

**Dossier zur Nutzenbewertung
gemäß § 35a SGB V**

Danicopan (Voydeya®)

Alexion Pharma Germany GmbH

**Modul 4 A, Anhang 4-G
Post-hoc Analysen Studie ALPHA**

*Zur Behandlung einer residualen hämolytischen Anämie
bei erwachsenen Patientinnen und Patienten mit
paroxysmaler nächtlicher Hämoglobinurie (PNH) als
Zusatztherapie zu Ravulizumab oder Eculizumab*

Dritter Datenschnitt vom 31.03.2023

Stand: 28.05.2024

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1 Morbidität

1.1 Hämoglobinwert-assoziierte Endpunkte

1.1.1 Veränderung des Hämoglobinwertes gegenüber Baseline

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	57		26		83	
Mean (SD)	76.7 (9.47)		79.0 (10.42)		77.4 (9.77)	
Median	78.0		80.5		78.0	
Min, Max	55, 94		54, 93		54, 94	
Week 1						
n	50	50	23	23		
Mean (SD)	98.6 (11.73)	21.4 (10.29)	88.1 (15.54)	9.8 (10.18)		
Median	100.0	20.0	84.0	10.0		
Min, Max	70, 122	-2, 45	62, 121	-5, 36		
Week 2						
n	55	55	23	23		
Mean (SD)	104.6 (11.94)	27.8 (12.32)	87.7 (13.38)	9.6 (9.28)		
Median	105.0	26.0	86.0	8.0		
Min, Max	80, 133	3, 55	64, 111	-9, 32		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	46	46	22	22		
Mean (SD)	105.5 (13.14)	29.0 (13.34)	87.8 (15.32)	10.3 (10.01)		
Median	105.0	28.0	88.5	7.5		
Min, Max	80, 126	-1, 59	58, 117	-3, 34		
Week 4						
n	52	52	24	24		
Mean (SD)	107.7 (15.93)	30.6 (13.57)	86.7 (14.10)	8.4 (9.69)		
Median	110.0	31.5	88.5	7.5		
Min, Max	74, 139	-7, 58	58, 114	-7, 30		
Week 6						
n	50	50	24	24		
Mean (SD)	106.8 (14.82)	30.0 (13.10)	86.5 (10.86)	8.2 (7.02)		
Median	107.5	28.5	87.5	8.0		
Min, Max	73, 133	2, 59	62, 107	-5, 19		

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	46	46	24	24		
Mean (SD)	103.7 (15.87)	27.2 (16.02)	87.1 (12.52)	8.6 (8.35)		
Median	103.0	27.0	89.0	9.5		
Min, Max	64, 130	-14, 68	66, 112	-9, 26		
Week 10						
n	38	38	22	22		
Mean (SD)	106.8 (14.12)	29.4 (13.40)	84.8 (14.19)	5.2 (9.36)		
Median	109.5	28.5	86.5	4.5		
Min, Max	80, 130	9, 54	51, 106	-20, 20		
Week 12						
n	47	47	24	24		
Mean (SD)	105.9 (15.06)	30.0 (14.92)	86.2 (10.98)	7.1 (8.62)		
Median	112.0	31.0	87.0	7.0		
Min, Max	68, 131	-8, 60	54, 106	-7, 31		

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	42	42	21	21		
Mean (SD)	106.4 (15.79)	29.7 (14.81)	96.6 (10.46)	18.2 (7.93)		
Median	105.5	28.0	99.0	17.0		
Min, Max	70, 135	-1, 59	75, 112	1, 32		
Week 14						
n	45	45	22	22		
Mean (SD)	106.6 (17.93)	30.1 (16.99)	106.9 (16.60)	27.1 (13.99)		
Median	110.0	32.0	108.0	25.5		
Min, Max	70, 146	-5, 69	81, 152	4, 72		
Week 15						
n	40	40	19	19		
Mean (SD)	105.6 (15.99)	28.7 (16.01)	107.1 (14.30)	27.4 (10.71)		
Median	108.0	29.0	109.0	29.0		
Min, Max	75, 145	-6, 64	85, 141	7, 50		

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	46	46	24	24		
Mean (SD)	106.1 (17.30)	28.6 (16.16)	105.2 (16.43)	26.6 (14.60)		
Median	106.0	28.5	105.0	27.0		
Min, Max	67, 147	-4, 66	74, 153	3, 60		
Week 18						
n	40	40	20	20		
Mean (SD)	101.3 (18.27)	25.0 (18.55)	103.1 (16.79)	23.4 (12.48)		
Median	99.5	24.5	104.5	24.5		
Min, Max	69, 143	-4, 63	73, 139	0, 46		
Week 20						
n	41	41	19	19		
Mean (SD)	105.4 (18.27)	28.2 (17.12)	101.8 (16.90)	21.4 (15.52)		
Median	109.0	29.0	100.0	19.0		
Min, Max	67, 145	-4, 64	66, 136	-5, 49		

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	43	43	19	19		
Mean (SD)	107.2 (19.38)	30.5 (19.48)	105.8 (16.71)	27.8 (16.02)		
Median	110.0	33.0	107.0	29.0		
Min, Max	65, 146	-6, 67	70, 143	-1, 53		
Week 24						
n	47	47	18	18		
Mean (SD)	105.9 (18.18)	28.9 (18.61)	104.4 (17.40)	26.1 (14.83)		
Median	104.0	30.0	105.0	27.5		
Min, Max	70, 152	-6, 71	76, 136	0, 51		
Week 28						
n	42	42	18	18	60	60
Mean (SD)	104.9 (19.34)	28.8 (18.68)	102.8 (17.54)	24.1 (16.24)	104.3 (18.70)	27.4 (17.98)
Median	103.0	28.5	101.5	22.5	103.0	23.5
Min, Max	60, 148	-16, 67	64, 131	-12, 50	60, 148	-16, 67

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Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	44	44	18	18	62	62
Mean (SD)	104.7 (17.74)	28.0 (18.77)	101.3 (15.71)	22.7 (14.41)	103.7 (17.12)	26.5 (17.67)
Median	105.5	27.5	100.0	21.5	103.5	25.0
Min, Max	66, 133	-9, 71	66, 132	-2, 48	66, 133	-9, 71
Week 36						
n	35	35	20	20	55	55
Mean (SD)	106.7 (17.53)	31.1 (17.00)	107.8 (13.23)	29.4 (15.64)	107.1 (15.98)	30.5 (16.39)
Median	110.0	32.0	106.5	30.5	109.0	31.0
Min, Max	73, 143	-3, 62	86, 124	8, 61	73, 143	-3, 62
Week 40						
n	37	37	18	18	55	55
Mean (SD)	106.4 (18.68)	31.0 (19.64)	103.7 (17.50)	25.2 (15.62)	105.5 (18.19)	29.1 (18.48)
Median	106.0	33.0	99.5	24.5	104.0	28.0
Min, Max	66, 155	-1, 74	57, 131	-5, 53	57, 155	-5, 74

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Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	37	37	16	16	53	53
Mean (SD)	106.5 (19.85)	30.2 (18.27)	109.2 (13.70)	32.1 (14.06)	107.3 (18.12)	30.8 (16.99)
Median	108.0	30.0	105.0	33.5	107.0	30.0
Min, Max	58, 146	-17, 73	89, 131	11, 55	58, 146	-17, 73
Week 48						
n	31	31	18	18	49	49
Mean (SD)	105.6 (18.51)	29.3 (18.32)	106.7 (14.68)	29.2 (13.21)	106.0 (17.05)	29.3 (16.48)
Median	104.0	29.0	111.0	31.5	108.0	31.0
Min, Max	68, 153	3, 72	82, 129	4, 50	68, 153	3, 72
Week 52						
n	27	27	16	16	43	43
Mean (SD)	107.4 (19.03)	31.4 (15.74)	105.9 (19.69)	28.3 (15.32)	106.9 (19.06)	30.3 (15.48)
Median	108.0	36.0	105.5	28.5	108.0	32.0
Min, Max	62, 143	-3, 62	64, 142	2, 53	62, 143	-3, 62

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	32	32	16	16	48	48
Mean (SD)	105.4 (20.72)	29.1 (17.83)	104.9 (15.84)	28.3 (14.73)	105.2 (19.06)	28.8 (16.71)
Median	107.5	30.0	106.5	27.0	107.0	29.5
Min, Max	52, 148	-5, 67	78, 128	-3, 54	52, 148	-5, 67
Week 60						
n	24	24	15	15	39	39
Mean (SD)	106.3 (20.12)	30.2 (17.92)	101.7 (17.83)	23.5 (15.28)	104.5 (19.16)	27.6 (17.06)
Median	103.5	32.0	99.0	22.0	102.0	30.0
Min, Max	60, 151	-2, 70	75, 141	4, 52	60, 151	-2, 70
Week 64						
n	21	21	14	14	35	35
Mean (SD)	104.5 (19.00)	29.6 (15.13)	102.3 (16.99)	25.5 (16.22)	103.6 (18.00)	28.0 (15.48)
Median	106.0	28.0	102.5	30.0	105.0	28.0
Min, Max	47, 137	-10, 56	72, 131	-2, 57	47, 137	-10, 57

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.6.2.1
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	19	19	12	12	31	31
Mean (SD)	104.6 (19.42)	29.5 (15.59)	100.3 (17.93)	23.2 (16.59)	102.9 (18.68)	27.0 (16.01)
Median	109.0	32.0	105.5	25.0	107.0	29.0
Min, Max	50, 129	-7, 59	66, 132	-3, 56	50, 132	-7, 59
Week 72						
n	16	16	9	9	25	25
Mean (SD)	104.8 (16.41)	29.9 (12.21)	98.3 (18.51)	20.2 (17.81)	102.4 (17.10)	26.4 (14.87)
Median	106.5	27.5	91.0	24.0	106.0	27.0
Min, Max	68, 135	8, 47	70, 124	-11, 40	68, 135	-11, 47
Week 80						
n	11	11	7	7	18	18
Mean (SD)	102.5 (17.17)	26.2 (16.99)	104.7 (19.09)	28.7 (11.44)	103.3 (17.42)	27.2 (14.75)
Median	96.0	19.0	112.0	32.0	104.5	29.0
Min, Max	84, 132	8, 59	67, 123	4, 39	67, 132	4, 59

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:49:32

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Table GERMANY.2.6.2.1
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	9	9	6	6	15	15
Mean (SD)	103.7 (19.42)	27.0 (14.38)	98.5 (12.90)	21.2 (8.42)	101.6 (16.78)	24.7 (12.34)
Median	113.0	27.0	101.0	20.5	104.0	22.0
Min, Max	72, 128	10, 55	76, 110	12, 34	72, 128	10, 55
Week 96						
n	5	5	4	4	9	9
Mean (SD)	112.4 (15.90)	36.8 (15.25)	105.5 (18.08)	29.8 (9.84)	109.3 (16.19)	33.7 (12.90)
Median	117.0	44.0	110.0	30.5	113.0	31.0
Min, Max	91, 128	18, 52	80, 122	17, 41	80, 128	17, 52
Week 104						
n	1	1	2	2	3	3
Mean (SD)	109.0 (NA)	31.0 (NA)	109.5 (7.78)	25.5 (9.19)	109.3 (5.51)	27.3 (7.23)
Median	109.0	31.0	109.5	25.5	109.0	31.0
Min, Max	109, 109	31, 31	104, 115	19, 32	104, 115	19, 32

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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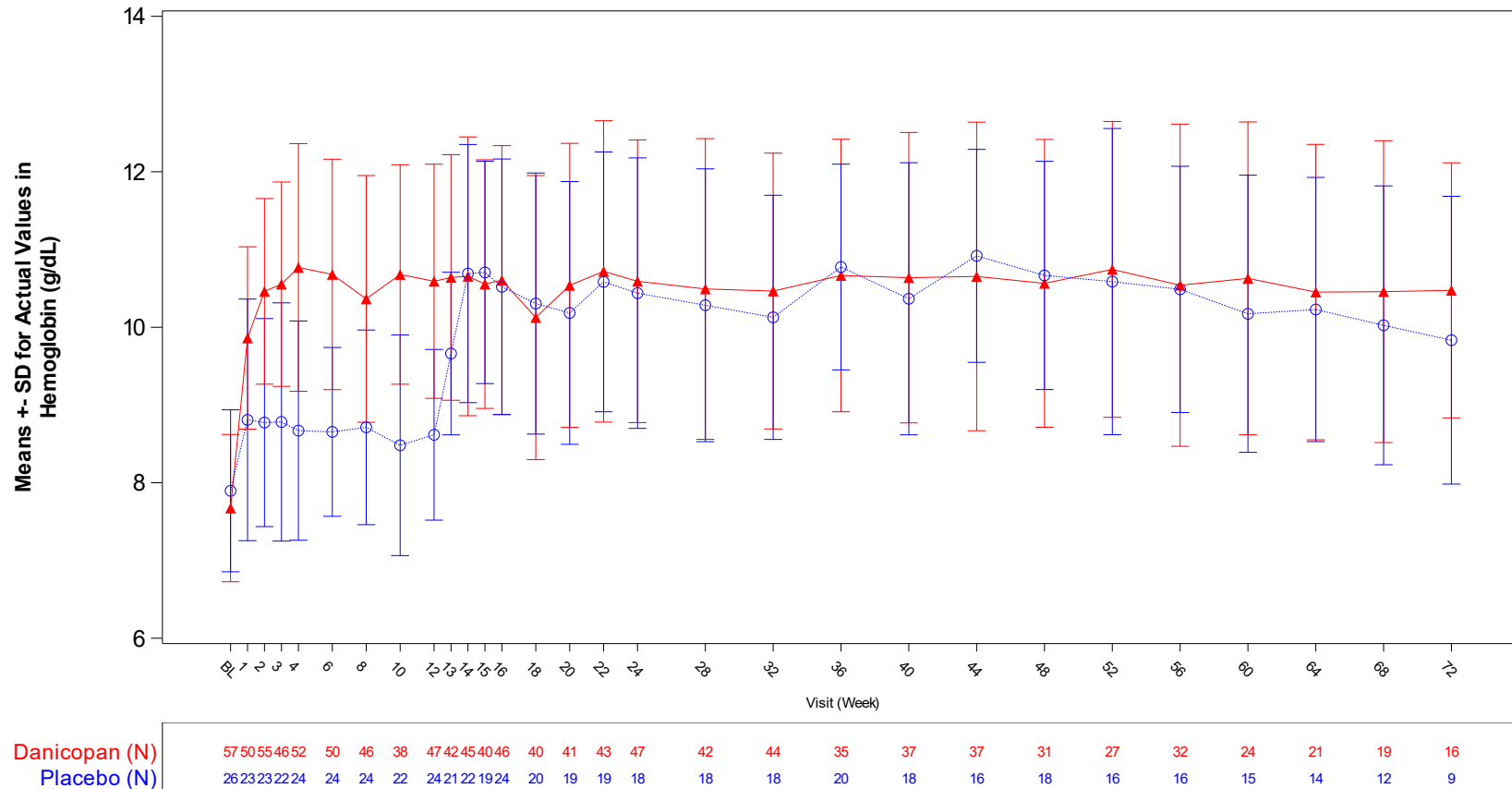
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Figure GERMANY.2.1.1.4.1
Plots of Mean \pm SD for Actual Values in Hemoglobin (g/dL) During 72 Weeks
Modified Randomized Set



Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Source: ADaM.ADLB

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Table GERMANY.2.1.1.1
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 25)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	20.90 (1.444)	8.65 (2.118)	12.25 (2.545)	<0.0001
	95% CI for LS MEAN	(18.02, 23.78)	(4.42, 12.88)	(7.17, 17.33)	
Week 2	LS Mean (SEM)	27.11 (1.566)	6.89 (2.391)	20.22 (2.841)	<0.0001
	95% CI for LS MEAN	(23.99, 30.23)	(2.13, 11.66)	(14.55, 25.88)	
Week 3	LS Mean (SEM)	29.14 (1.718)	5.95 (2.706)	23.19 (3.190)	<0.0001
	95% CI for LS MEAN	(25.71, 32.57)	(0.56, 11.34)	(16.83, 29.55)	
Week 4	LS Mean (SEM)	30.51 (2.020)	4.56 (3.319)	25.95 (3.873)	<0.0001
	95% CI for LS MEAN	(26.47, 34.55)	(-2.06, 11.18)	(18.22, 33.68)	
Week 6	LS Mean (SEM)	30.12 (1.883)	2.95 (3.046)	27.17 (3.571)	<0.0001
	95% CI for LS MEAN	(26.35, 33.89)	(-3.13, 9.03)	(20.04, 34.30)	
Week 8	LS Mean (SEM)	28.24 (2.059)	4.77 (3.313)	23.47 (3.889)	<0.0001
	95% CI for LS MEAN	(24.13, 32.36)	(-1.83, 11.38)	(15.71, 31.23)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening). An Unstructured covariance structure was used.

P-value is from difference of LSM from MMRM model.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Data cutoff date of 31Mar2023

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Table GERMANY.2.1.1.1
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 25)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	28.20 (2.016)	4.70 (3.179)	23.50 (3.749)	<0.0001
	95% CI for LS MEAN	(24.16, 32.23)	(-1.66, 11.06)	(16.00, 31.00)	
Week 12	LS Mean (SEM)	28.14 (1.964)	4.13 (3.085)	24.02 (3.643)	<0.0001
	95% CI for LS MEAN	(24.22, 32.06)	(-2.03, 10.28)	(16.75, 31.29)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening). An Unstructured covariance structure was used.

P-value is from difference of LSM from MMRM model.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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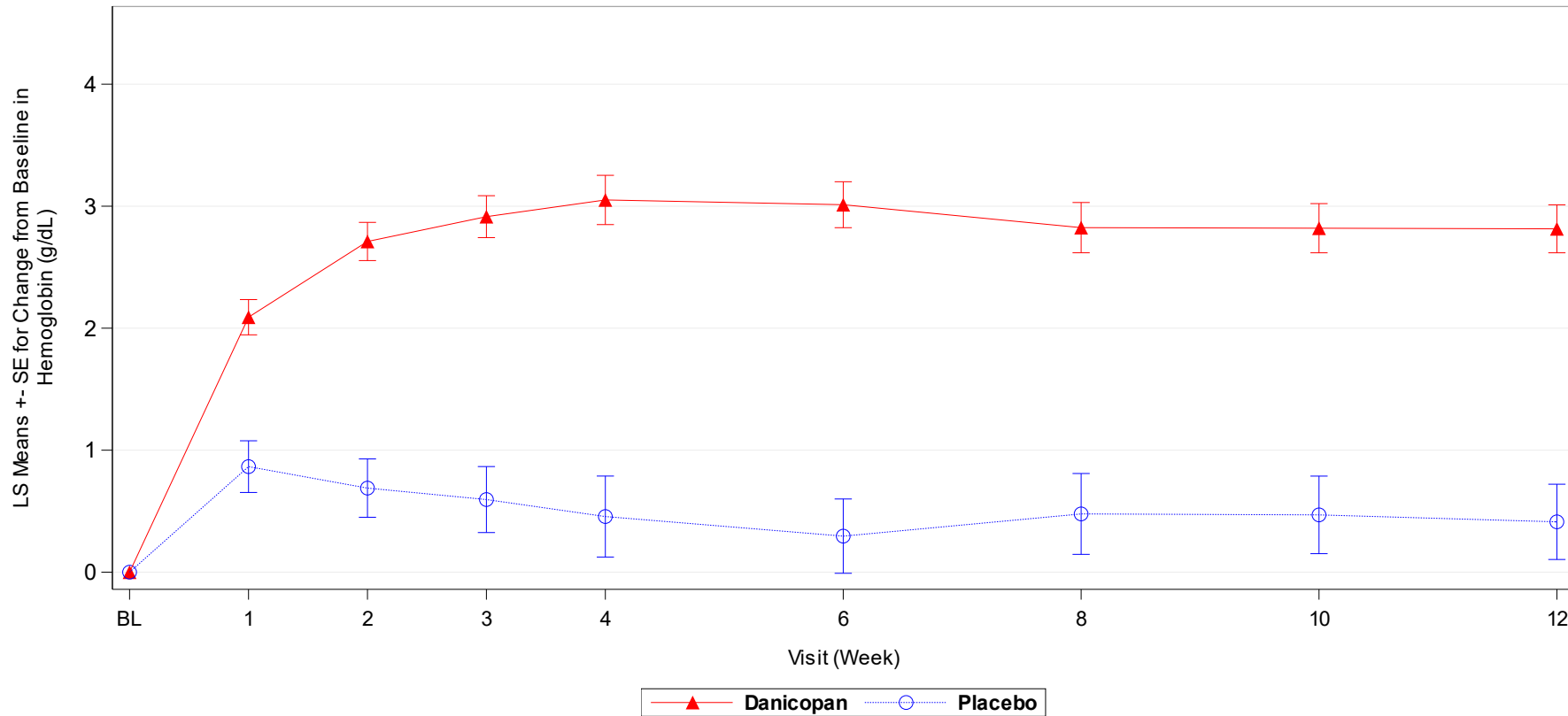
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Figure GERMANY.2.1.1.1.1
Plots of LS Means \pm SE for Change from Baseline in Hemoglobin (g/dL) During 12-Week of Treatment Period 1
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

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Data cutoff date of 31Mar2023

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.1 Hämoglobinwert-assoziierte Endpunkte

1.1.1 Veränderung des Hämoglobinwertes gegenüber Baseline

1.1.1.1 Subgruppenanalysen – Veränderung des Hämoglobinwertes gegenüber Baseline

Post-hoc Analysen Studie ALPHA

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Table GERMANY.2.6.2.2
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean (SD)	76.6 (7.43)		78.7 (13.62)		77.2 (9.38)	
Median	77.0		80.0		78.5	
Min, Max	59, 88		54, 93		54, 93	
Week 1						
n	22	22	9	9		
Mean (SD)	96.8 (10.58)	19.8 (9.49)	88.6 (19.82)	9.9 (11.53)		
Median	94.5	19.0	94.0	9.0		
Min, Max	79, 120	-2, 43	62, 114	-5, 36		
Week 2						
n	23	23	8	8		
Mean (SD)	103.4 (12.12)	26.8 (12.16)	93.4 (15.63)	14.9 (10.32)		
Median	104.0	26.0	97.0	13.0		
Min, Max	85, 126	4, 54	64, 111	3, 32		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.6.2.2
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	21	21	7	7		
Mean (SD)	106.3 (12.63)	29.7 (12.64)	90.1 (18.02)	13.6 (12.82)		
Median	104.0	30.0	90.0	7.0		
Min, Max	84, 126	1, 58	58, 117	-3, 34		
Week 4						
n	21	21	8	8		
Mean (SD)	105.2 (16.22)	28.3 (13.02)	88.1 (18.11)	9.6 (11.39)		
Median	106.0	28.0	88.5	7.0		
Min, Max	74, 130	5, 49	60, 114	-2, 30		
Week 6						
n	21	21	8	8		
Mean (SD)	107.8 (14.21)	31.0 (11.91)	87.9 (14.49)	9.4 (9.58)		
Median	109.0	28.0	89.0	13.0		
Min, Max	80, 133	8, 52	62, 107	-5, 19		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.6.2.2
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	18	18	9	9		
Mean (SD)	102.4 (14.64)	25.7 (14.60)	86.0 (13.93)	7.3 (6.75)		
Median	102.5	22.5	92.0	6.0		
Min, Max	78, 129	6, 48	66, 106	0, 18		
Week 10						
n	17	17	7	7		
Mean (SD)	107.6 (14.23)	30.8 (13.01)	86.6 (15.34)	4.3 (6.58)		
Median	111.0	30.0	87.0	3.0		
Min, Max	81, 128	12, 54	62, 106	-4, 15		
Week 12						
n	22	22	9	9		
Mean (SD)	107.5 (13.74)	30.6 (12.36)	86.9 (14.14)	8.2 (10.39)		
Median	109.0	28.5	89.0	8.0		
Min, Max	77, 131	10, 51	54, 100	-7, 31		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:49:33

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Table GERMANY.2.6.2.2
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	17	17	6	6		
Mean (SD)	108.4 (15.34)	32.0 (13.04)	97.0 (13.43)	18.8 (6.97)		
Median	107.0	31.0	97.0	16.0		
Min, Max	87, 135	13, 54	76, 112	14, 32		
Week 14						
n	19	19	8	8		
Mean (SD)	109.9 (17.52)	32.8 (15.72)	117.9 (19.81)	36.1 (17.12)		
Median	112.0	33.0	115.0	29.0		
Min, Max	70, 146	6, 65	89, 152	19, 72		
Week 15						
n	19	19	7	7		
Mean (SD)	108.4 (16.91)	31.2 (16.15)	112.6 (16.15)	31.0 (9.04)		
Median	111.0	31.0	110.0	30.0		
Min, Max	80, 145	-6, 64	87, 141	18, 48		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.6.2.2
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	21	21	9	9		
Mean (SD)	107.0 (16.60)	30.1 (14.75)	112.9 (16.99)	34.2 (15.18)		
Median	106.0	29.0	110.0	29.0		
Min, Max	78, 147	9, 66	93, 153	18, 60		
Week 18						
n	16	16	8	8		
Mean (SD)	103.7 (20.20)	27.2 (19.19)	108.9 (15.40)	27.1 (10.89)		
Median	102.0	25.5	104.5	29.5		
Min, Max	73, 143	-4, 62	92, 139	11, 46		
Week 20						
n	17	17	8	8		
Mean (SD)	108.0 (17.93)	31.6 (14.77)	109.0 (13.07)	27.3 (14.63)		
Median	112.0	32.0	107.5	28.0		
Min, Max	76, 145	2, 64	94, 136	2, 48		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:49:33

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Table GERMANY.2.6.2.2
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	18	18	8	8		
Mean (SD)	112.3 (18.49)	34.8 (16.71)	111.3 (15.29)	34.3 (14.96)		
Median	120.0	40.5	108.0	32.0		
Min, Max	81, 146	11, 65	93, 143	10, 53		
Week 24						
n	22	22	8	8		
Mean (SD)	111.1 (17.55)	34.1 (16.48)	105.9 (19.53)	28.9 (13.96)		
Median	112.5	31.5	105.5	30.5		
Min, Max	80, 152	3, 71	76, 136	0, 44		
Week 28						
n	18	18	7	7	25	25
Mean (SD)	105.3 (18.27)	28.9 (17.10)	104.1 (19.40)	27.3 (19.91)	105.0 (18.19)	28.5 (17.52)
Median	104.0	29.0	104.0	29.0	104.0	29.0
Min, Max	67, 148	0, 67	71, 131	-12, 50	67, 148	-12, 67

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Source: ADaM.ADSL, ADaM.ADLB

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Run Date: 2024-04-26T15:49:33

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Table GERMANY.2.6.2.2
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	19	19	7	7	26	26
Mean (SD)	107.3 (15.16)	30.4 (13.60)	96.9 (17.72)	20.4 (14.91)	104.5 (16.22)	27.7 (14.39)
Median	108.0	29.0	105.0	20.0	106.0	27.5
Min, Max	84, 132	9, 58	66, 118	-2, 45	66, 132	-2, 58
Week 36						
n	16	16	7	7	23	23
Mean (SD)	109.3 (17.83)	33.5 (15.02)	107.4 (12.70)	30.9 (17.10)	108.7 (16.17)	32.7 (15.34)
Median	109.5	30.5	107.0	31.0	109.0	31.0
Min, Max	82, 143	5, 62	90, 122	10, 55	82, 143	5, 62
Week 40						
n	16	16	6	6	22	22
Mean (SD)	109.1 (19.62)	33.4 (20.23)	109.2 (14.27)	32.8 (13.32)	109.1 (17.98)	33.2 (18.29)
Median	107.0	30.5	108.0	33.0	107.0	32.5
Min, Max	76, 155	-1, 74	93, 127	13, 53	76, 155	-1, 74

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Table GERMANY.2.6.2.2
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	17	17	6	6	23	23
Mean (SD)	108.5 (19.62)	32.5 (18.36)	108.0 (12.07)	32.5 (8.98)	108.3 (17.70)	32.5 (16.23)
Median	108.0	30.0	103.5	31.0	107.0	30.0
Min, Max	73, 146	-3, 73	96, 125	21, 44	73, 146	-3, 73
Week 48						
n	16	16	6	6	22	22
Mean (SD)	108.8 (19.62)	32.4 (19.57)	110.7 (14.76)	35.2 (7.63)	109.3 (18.10)	33.2 (16.99)
Median	106.5	34.0	111.0	32.5	109.5	33.5
Min, Max	84, 153	7, 72	85, 129	29, 50	84, 153	7, 72
Week 52						
n	13	13	5	5	18	18
Mean (SD)	109.7 (18.91)	32.5 (15.10)	106.8 (22.11)	31.6 (13.18)	108.9 (19.22)	32.3 (14.21)
Median	109.0	32.0	105.0	29.0	107.5	30.5
Min, Max	80, 143	9, 62	81, 142	14, 49	80, 143	9, 62

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Table GERMANY.2.6.2.2
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Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	15	15	4	4	19	19
Mean (SD)	109.7 (20.60)	34.9 (17.63)	97.8 (11.53)	27.0 (15.51)	107.2 (19.42)	33.2 (17.11)
Median	113.0	33.0	101.5	27.0	107.0	33.0
Min, Max	74, 148	4, 67	81, 107	8, 46	74, 148	4, 67
Week 60						
n	12	12	4	4	16	16
Mean (SD)	108.8 (19.63)	32.2 (19.04)	105.3 (24.74)	26.5 (17.79)	107.9 (20.19)	30.8 (18.32)
Median	103.5	31.5	98.0	26.5	100.5	30.5
Min, Max	90, 151	7, 70	84, 141	5, 48	84, 151	5, 70
Week 64						
n	11	11	3	3	14	14
Mean (SD)	106.9 (13.23)	30.2 (11.51)	98.3 (14.01)	24.3 (15.31)	105.1 (13.35)	28.9 (12.01)
Median	103.0	28.0	99.0	30.0	103.0	28.0
Min, Max	91, 137	16, 56	84, 112	7, 36	84, 137	7, 56

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Table GERMANY.2.6.2.2
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	7	7	3	3	10	10
Mean (SD)	107.0 (15.17)	30.1 (11.95)	101.3 (8.33)	27.3 (13.61)	105.3 (13.28)	29.3 (11.76)
Median	108.0	28.0	104.0	32.0	106.0	30.0
Min, Max	90, 129	14, 48	92, 108	12, 38	90, 129	12, 48
Week 72						
n	7	7	1	1	8	8
Mean (SD)	108.3 (18.25)	30.7 (14.30)	109.0 (NA)	33.0 (NA)	108.4 (16.89)	31.0 (13.27)
Median	107.0	27.0	109.0	33.0	108.0	30.0
Min, Max	84, 135	8, 47	109, 109	33, 33	84, 135	8, 47
Week 80						
n	6	6	1	1	7	7
Mean (SD)	103.7 (17.67)	27.2 (15.79)	105.0 (NA)	29.0 (NA)	103.9 (16.14)	27.4 (14.43)
Median	102.5	25.5	105.0	29.0	105.0	29.0
Min, Max	84, 132	8, 51	105, 105	29, 29	84, 132	8, 51

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Table GERMANY.2.6.2.2
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	4	4	1	1	5	5
Mean (SD)	103.8 (13.79)	25.0 (7.66)	98.0 (NA)	22.0 (NA)	102.6 (12.22)	24.4 (6.77)
Median	105.0	23.0	98.0	22.0	98.0	22.0
Min, Max	89, 116	19, 35	98, 98	22, 22	89, 116	19, 35
Week 96						
n	2	2	1	1	3	3
Mean (SD)	122.5 (7.78)	45.5 (2.12)	107.0 (NA)	31.0 (NA)	117.3 (10.50)	40.7 (8.50)
Median	122.5	45.5	107.0	31.0	117.0	44.0
Min, Max	117, 128	44, 47	107, 107	31, 31	107, 128	31, 47

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Table GERMANY.2.6.2.2
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		17		51	
Mean (SD)	76.8 (10.75)		79.1 (8.76)		77.6 (10.10)	
Median	78.0		81.0		78.0	
Min, Max	55, 94		62, 93		55, 94	
Week 1						
n	28	28	14	14		
Mean (SD)	100.0 (12.57)	22.6 (10.88)	87.8 (12.90)	9.7 (9.67)		
Median	102.5	22.0	82.5	10.5		
Min, Max	70, 122	2, 45	74, 121	-5, 35		
Week 2						
n	32	32	15	15		
Mean (SD)	105.5 (11.93)	28.6 (12.58)	84.7 (11.46)	6.8 (7.60)		
Median	105.5	27.0	83.0	7.0		
Min, Max	80, 133	3, 55	70, 109	-9, 20		

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Table GERMANY.2.6.2.2
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
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Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	25	25	15	15		
Mean (SD)	104.9 (13.79)	28.3 (14.14)	86.7 (14.46)	8.8 (8.50)		
Median	107.0	27.0	84.0	8.0		
Min, Max	80, 123	-1, 59	64, 116	-3, 31		
Week 4						
n	31	31	16	16		
Mean (SD)	109.4 (15.78)	32.2 (13.92)	86.0 (12.26)	7.8 (9.07)		
Median	111.0	32.0	88.5	7.5		
Min, Max	74, 139	-7, 58	58, 103	-7, 25		
Week 6						
n	29	29	16	16		
Mean (SD)	106.1 (15.46)	29.2 (14.06)	85.9 (9.02)	7.6 (5.62)		
Median	107.0	29.0	86.5	7.5		
Min, Max	73, 127	2, 59	70, 101	-2, 18		

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Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	28	28	15	15		
Mean (SD)	104.4 (16.83)	28.1 (17.06)	87.8 (12.06)	9.4 (9.32)		
Median	103.0	31.5	89.0	10.0		
Min, Max	64, 130	-14, 68	72, 112	-9, 26		
Week 10						
n	21	21	15	15		
Mean (SD)	106.1 (14.35)	28.2 (13.91)	84.0 (14.10)	5.6 (10.60)		
Median	109.0	28.0	86.0	8.0		
Min, Max	80, 130	9, 54	51, 105	-20, 20		
Week 12						
n	25	25	15	15		
Mean (SD)	104.5 (16.29)	29.6 (17.11)	85.7 (9.13)	6.5 (7.70)		
Median	113.0	31.0	86.0	5.0		
Min, Max	68, 124	-8, 60	69, 106	-5, 21		

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
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Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	25	25	15	15		
Mean (SD)	105.0 (16.26)	28.1 (15.96)	96.5 (9.58)	17.9 (8.50)		
Median	102.0	27.0	99.0	18.0		
Min, Max	70, 129	-1, 59	75, 109	1, 31		
Week 14						
n	26	26	14	14		
Mean (SD)	104.1 (18.18)	28.0 (17.89)	100.6 (10.85)	21.9 (8.92)		
Median	106.0	32.0	102.0	24.0		
Min, Max	70, 131	-5, 69	81, 114	4, 38		
Week 15						
n	21	21	12	12		
Mean (SD)	103.0 (15.06)	26.4 (15.91)	103.8 (12.71)	25.3 (11.39)		
Median	100.0	27.0	102.0	24.0		
Min, Max	75, 125	2, 62	85, 126	7, 50		

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Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	25	25	15	15		
Mean (SD)	105.2 (18.17)	27.3 (17.45)	100.6 (14.77)	22.1 (12.61)		
Median	106.0	26.0	95.0	23.0		
Min, Max	67, 137	-4, 63	74, 120	3, 44		
Week 18						
n	24	24	12	12		
Mean (SD)	99.6 (17.12)	23.5 (18.38)	99.2 (17.17)	20.8 (13.28)		
Median	99.5	23.5	103.5	20.0		
Min, Max	69, 127	-2, 63	73, 120	0, 42		
Week 20						
n	24	24	11	11		
Mean (SD)	103.5 (18.66)	25.9 (18.55)	96.6 (18.00)	17.2 (15.39)		
Median	102.5	28.0	99.0	12.0		
Min, Max	67, 133	-4, 54	66, 125	-5, 49		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.6.2.2
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	25	25	11	11		
Mean (SD)	103.5 (19.55)	27.4 (21.03)	101.9 (17.29)	23.1 (15.74)		
Median	107.0	31.0	97.0	25.0		
Min, Max	65, 131	-6, 67	70, 125	-1, 46		
Week 24						
n	25	25	10	10		
Mean (SD)	101.3 (17.81)	24.4 (19.50)	103.2 (16.48)	23.9 (15.86)		
Median	100.0	19.0	99.0	21.0		
Min, Max	70, 142	-6, 61	79, 129	3, 51		
Week 28						
n	24	24	11	11	35	35
Mean (SD)	104.7 (20.49)	28.6 (20.15)	102.0 (17.19)	22.1 (14.12)	103.8 (19.30)	26.6 (18.51)
Median	102.0	24.5	101.0	18.0	101.0	20.0
Min, Max	60, 142	-16, 62	64, 129	2, 45	60, 142	-16, 62

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.2
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	25	25	11	11	36	36
Mean (SD)	102.7 (19.54)	26.2 (22.02)	104.1 (14.44)	24.2 (14.61)	103.1 (17.94)	25.6 (19.86)
Median	102.0	25.0	99.0	23.0	101.5	23.5
Min, Max	66, 133	-9, 71	86, 132	8, 48	66, 133	-9, 71
Week 36						
n	19	19	13	13	32	32
Mean (SD)	104.4 (17.43)	29.2 (18.66)	107.9 (14.01)	28.6 (15.46)	105.8 (15.98)	28.9 (17.17)
Median	111.0	34.0	106.0	30.0	108.5	32.0
Min, Max	73, 127	-3, 56	86, 124	8, 61	73, 127	-3, 61
Week 40						
n	21	21	12	12	33	33
Mean (SD)	104.3 (18.14)	29.1 (19.47)	100.9 (18.87)	21.3 (15.76)	103.1 (18.18)	26.3 (18.35)
Median	105.0	33.0	98.0	18.0	103.0	24.0
Min, Max	66, 136	-1, 65	57, 131	-5, 47	57, 136	-5, 65

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Table GERMANY.2.6.2.2
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	20	20	10	10	30	30
Mean (SD)	104.9 (20.40)	28.4 (18.45)	109.9 (15.18)	31.9 (16.86)	106.6 (18.70)	29.5 (17.72)
Median	107.5	31.0	110.5	35.5	107.5	34.5
Min, Max	58, 134	-17, 55	89, 131	11, 55	58, 134	-17, 55
Week 48						
n	15	15	12	12	27	27
Mean (SD)	102.3 (17.29)	26.0 (16.91)	104.7 (14.86)	26.2 (14.63)	103.4 (15.99)	26.1 (15.64)
Median	103.0	25.0	106.5	28.5	103.0	28.0
Min, Max	68, 127	3, 53	82, 127	4, 49	68, 127	3, 53
Week 52						
n	14	14	11	11	25	25
Mean (SD)	105.4 (19.60)	30.4 (16.81)	105.5 (19.63)	26.7 (16.56)	105.4 (19.20)	28.8 (16.46)
Median	107.5	37.0	113.0	28.0	108.0	33.0
Min, Max	62, 130	-3, 56	64, 127	2, 53	62, 130	-3, 56

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.2
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	17	17	12	12	29	29
Mean (SD)	101.6 (20.70)	24.0 (16.89)	107.3 (16.77)	28.8 (15.15)	104.0 (19.06)	26.0 (16.09)
Median	98.0	23.0	111.5	29.0	108.0	26.0
Min, Max	52, 128	-5, 53	78, 128	-3, 54	52, 128	-5, 54
Week 60						
n	12	12	11	11	23	23
Mean (SD)	103.8 (21.15)	28.2 (17.31)	100.5 (15.95)	22.4 (15.06)	102.2 (18.49)	25.4 (16.18)
Median	108.5	32.5	103.0	22.0	103.0	29.0
Min, Max	60, 125	-2, 52	75, 126	4, 52	60, 126	-2, 52
Week 64						
n	10	10	11	11	21	21
Mean (SD)	101.9 (24.35)	29.0 (18.99)	103.4 (18.17)	25.8 (17.17)	102.7 (20.80)	27.3 (17.67)
Median	106.5	31.5	105.0	30.0	106.0	30.0
Min, Max	47, 131	-10, 56	72, 131	-2, 57	47, 131	-10, 57

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.2
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	12	12	9	9	21	21
Mean (SD)	103.2 (22.04)	29.1 (17.87)	99.9 (20.60)	21.8 (17.98)	101.8 (20.97)	26.0 (17.85)
Median	113.0	35.0	107.0	24.0	107.0	29.0
Min, Max	50, 122	-7, 59	66, 132	-3, 56	50, 132	-7, 59
Week 72						
n	9	9	8	8	17	17
Mean (SD)	102.0 (15.35)	29.2 (11.18)	97.0 (19.32)	18.6 (18.34)	99.6 (16.96)	24.2 (15.47)
Median	106.0	28.0	91.0	22.5	105.0	27.0
Min, Max	68, 119	11, 46	70, 124	-11, 40	68, 124	-11, 46
Week 80						
n	5	5	6	6	11	11
Mean (SD)	101.0 (18.49)	25.0 (20.16)	104.7 (20.92)	28.7 (12.53)	103.0 (18.95)	27.0 (15.65)
Median	92.0	19.0	113.0	32.5	104.0	29.0
Min, Max	87, 132	8, 59	67, 123	4, 39	67, 132	4, 59

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.2
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	5	5	5	5	10	10
Mean (SD)	103.6 (24.72)	28.6 (19.03)	98.6 (14.42)	21.0 (9.41)	101.1 (19.26)	24.8 (14.71)
Median	113.0	33.0	104.0	19.0	107.0	23.0
Min, Max	72, 128	10, 55	76, 110	12, 34	72, 128	10, 55
Week 96						
n	3	3	3	3	6	6
Mean (SD)	105.7 (17.47)	31.0 (18.36)	105.0 (22.11)	29.3 (12.01)	105.3 (17.83)	30.2 (13.91)
Median	101.0	23.0	113.0	30.0	107.0	26.5
Min, Max	91, 125	18, 52	80, 122	17, 41	80, 125	17, 52
Week 104						
n	1	1	2	2	3	3
Mean (SD)	109.0 (NA)	31.0 (NA)	109.5 (7.78)	25.5 (9.19)	109.3 (5.51)	27.3 (7.23)
Median	109.0	31.0	109.5	25.5	109.0	31.0
Min, Max	109, 109	31, 31	104, 115	19, 32	104, 115	19, 32

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.6.2.3

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	41		20		61	
Mean (SD)	76.9 (9.91)		77.9 (11.06)		77.2 (10.21)	
Median	79.0		79.5		79.0	
Min, Max	55, 94		54, 93		54, 94	
Week 1						
n	35	35	18	18		
Mean (SD)	96.6 (12.23)	19.0 (9.71)	87.1 (16.75)	10.4 (11.35)		
Median	96.0	19.0	82.5	10.5		
Min, Max	70, 122	-2, 38	62, 121	-5, 36		
Week 2						
n	41	41	18	18		
Mean (SD)	103.2 (12.02)	26.4 (12.32)	86.5 (13.87)	9.8 (10.34)		
Median	103.0	24.0	84.5	8.0		
Min, Max	80, 133	3, 55	64, 111	-9, 32		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.3

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	32	32	18	18		
Mean (SD)	104.7 (13.96)	28.1 (14.63)	88.1 (16.53)	11.3 (10.77)		
Median	104.5	28.0	88.5	10.0		
Min, Max	80, 126	-1, 59	58, 117	-3, 34		
Week 4						
n	37	37	19	19		
Mean (SD)	107.5 (16.70)	30.1 (14.21)	87.0 (15.13)	9.9 (9.61)		
Median	108.0	31.0	87.0	9.0		
Min, Max	74, 139	-7, 58	58, 114	-5, 30		
Week 6						
n	35	35	19	19		
Mean (SD)	107.1 (15.24)	29.7 (13.12)	85.8 (11.76)	8.7 (6.39)		
Median	109.0	28.0	87.0	8.0		
Min, Max	73, 133	2, 52	62, 107	-2, 19		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Table GERMANY.2.6.2.3

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	31	31	18	18		
Mean (SD)	104.5 (14.64)	27.5 (14.07)	86.8 (13.98)	9.7 (8.86)		
Median	103.0	28.0	86.0	10.0		
Min, Max	78, 130	5, 48	66, 112	-9, 26		
Week 10						
n	28	28	16	16		
Mean (SD)	107.9 (14.69)	29.8 (13.64)	83.9 (15.51)	5.4 (10.30)		
Median	110.5	29.5	85.5	6.5		
Min, Max	80, 130	9, 54	51, 105	-20, 20		
Week 12						
n	35	35	18	18		
Mean (SD)	107.2 (14.87)	30.9 (14.31)	85.8 (11.14)	8.0 (9.27)		
Median	113.0	31.0	86.5	7.0		
Min, Max	77, 131	3, 60	54, 106	-7, 31		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.3

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	32	32	16	16		
Mean (SD)	106.9 (16.96)	30.1 (16.07)	95.8 (10.81)	18.1 (8.55)		
Median	109.5	27.5	99.0	17.0		
Min, Max	70, 135	-1, 59	75, 110	1, 32		
Week 14						
n	32	32	16	16		
Mean (SD)	107.9 (18.55)	31.5 (17.81)	103.3 (14.47)	24.5 (10.73)		
Median	110.0	32.0	102.0	25.0		
Min, Max	70, 146	3, 69	81, 137	4, 45		
Week 15						
n	28	28	14	14		
Mean (SD)	107.7 (16.51)	30.4 (16.06)	105.0 (15.06)	26.4 (10.44)		
Median	112.0	30.0	104.5	27.5		
Min, Max	75, 145	4, 64	85, 141	7, 48		

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.3

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	33	33	18	18		
Mean (SD)	107.8 (18.25)	30.0 (17.20)	102.9 (18.10)	25.7 (16.19)		
Median	106.0	30.0	99.0	27.0		
Min, Max	67, 147	-4, 66	74, 153	3, 60		
Week 18						
n	29	29	14	14		
Mean (SD)	101.3 (19.31)	24.3 (19.76)	99.8 (18.51)	21.4 (12.65)		
Median	99.0	23.0	98.0	21.5		
Min, Max	69, 143	-4, 63	73, 139	0, 46		
Week 20						
n	31	31	14	14		
Mean (SD)	104.7 (19.11)	27.6 (17.64)	99.6 (18.10)	20.5 (15.32)		
Median	105.0	29.0	99.5	16.5		
Min, Max	67, 145	-4, 64	66, 136	-5, 48		

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Table GERMANY.2.6.2.3

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	31	31	15	15		
Mean (SD)	107.8 (20.10)	30.8 (20.45)	104.7 (17.80)	27.7 (16.84)		
Median	109.0	32.0	106.0	29.0		
Min, Max	65, 146	-6, 67	70, 143	-1, 53		
Week 24						
n	34	34	14	14		
Mean (SD)	106.9 (18.79)	29.7 (18.85)	103.0 (17.29)	25.8 (13.59)		
Median	104.0	30.5	104.0	27.5		
Min, Max	70, 152	-4, 71	76, 136	3, 45		
Week 28						
n	31	31	14	14	45	45
Mean (SD)	105.6 (19.63)	29.0 (18.15)	103.0 (17.09)	25.2 (14.91)	104.8 (18.72)	27.8 (17.13)
Median	103.0	28.0	100.0	21.0	102.0	22.0
Min, Max	67, 148	0, 67	64, 131	2, 50	64, 148	0, 67

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	32	32	14	14	46	46
Mean (SD)	103.9 (18.25)	27.4 (19.24)	99.8 (15.26)	23.1 (12.90)	102.6 (17.33)	26.1 (17.53)
Median	103.5	24.5	99.0	21.5	101.5	23.0
Min, Max	66, 133	-9, 71	66, 132	8, 48	66, 133	-9, 71
Week 36						
n	22	22	17	17	39	39
Mean (SD)	107.5 (18.04)	32.5 (16.37)	106.9 (12.97)	29.4 (15.66)	107.3 (15.84)	31.2 (15.93)
Median	109.5	32.0	106.0	30.0	107.0	31.0
Min, Max	78, 143	-1, 62	86, 124	8, 61	78, 143	-1, 62
Week 40						
n	26	26	14	14	40	40
Mean (SD)	107.0 (20.32)	31.5 (21.29)	101.9 (18.59)	24.1 (16.51)	105.2 (19.64)	28.9 (19.85)
Median	106.5	33.0	98.0	23.0	103.5	27.5
Min, Max	66, 155	-1, 74	57, 131	-5, 53	57, 155	-5, 74

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	25	25	14	14	39	39
Mean (SD)	106.0 (20.28)	29.8 (18.46)	106.9 (13.04)	30.6 (13.60)	106.4 (17.83)	30.1 (16.69)
Median	107.0	30.0	103.5	33.5	105.0	32.0
Min, Max	58, 146	-17, 65	89, 131	11, 53	58, 146	-17, 65
Week 48						
n	22	22	15	15	37	37
Mean (SD)	103.4 (19.26)	27.0 (17.85)	104.2 (14.81)	27.3 (13.21)	103.7 (17.37)	27.1 (15.93)
Median	103.0	26.5	108.0	31.0	104.0	29.0
Min, Max	68, 153	3, 72	82, 129	4, 50	68, 153	3, 72
Week 52						
n	18	18	14	14	32	32
Mean (SD)	107.5 (21.79)	32.5 (17.44)	104.4 (20.26)	27.5 (14.40)	106.1 (20.86)	30.3 (16.13)
Median	111.0	37.0	103.5	28.5	108.5	32.5
Min, Max	62, 143	-3, 62	64, 142	2, 49	62, 143	-3, 62

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	23	23	13	13	36	36
Mean (SD)	104.4 (23.37)	28.1 (19.82)	102.6 (15.85)	27.0 (12.99)	103.8 (20.74)	27.7 (17.46)
Median	109.0	30.0	106.0	27.0	106.5	28.0
Min, Max	52, 148	-5, 67	78, 125	-3, 46	52, 148	-5, 67
Week 60						
n	15	15	12	12	27	27
Mean (SD)	105.2 (22.46)	29.4 (18.73)	99.5 (18.40)	21.8 (13.45)	102.7 (20.57)	26.0 (16.73)
Median	105.0	33.0	95.0	22.0	102.0	29.0
Min, Max	60, 151	-2, 70	75, 141	4, 48	60, 151	-2, 70
Week 64						
n	15	15	11	11	26	26
Mean (SD)	101.7 (20.62)	27.4 (15.79)	99.1 (16.46)	23.4 (13.88)	100.6 (18.66)	25.7 (14.86)
Median	103.0	28.0	100.0	30.0	103.0	28.0
Min, Max	47, 137	-10, 56	72, 123	-2, 39	47, 137	-10, 56

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	13	13	10	10	23	23
Mean (SD)	104.4 (22.69)	29.8 (18.03)	96.7 (16.30)	21.0 (14.06)	101.0 (20.12)	26.0 (16.67)
Median	113.0	34.0	99.5	25.0	107.0	29.0
Min, Max	50, 129	-7, 59	66, 116	-3, 38	50, 129	-7, 59
Week 72						
n	12	12	9	9	21	21
Mean (SD)	108.2 (17.10)	32.3 (12.30)	98.3 (18.51)	20.2 (17.81)	104.0 (17.96)	27.1 (15.74)
Median	109.5	33.0	91.0	24.0	109.0	29.0
Min, Max	68, 135	11, 47	70, 124	-11, 40	68, 135	-11, 47
Week 80						
n	8	8	7	7	15	15
Mean (SD)	108.0 (17.01)	29.4 (18.60)	104.7 (19.09)	28.7 (11.44)	106.5 (17.43)	29.1 (15.14)
Median	106.5	25.5	112.0	32.0	109.0	32.0
Min, Max	87, 132	8, 59	67, 123	4, 39	67, 132	4, 59

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	5	5	5	5	10	10
Mean (SD)	102.8 (23.97)	27.4 (18.88)	96.2 (12.97)	18.6 (6.27)	99.5 (18.50)	23.0 (14.05)
Median	115.0	27.0	98.0	19.0	101.0	20.5
Min, Max	72, 128	10, 55	76, 110	12, 27	72, 128	10, 55
Week 96						
n	4	4	4	4	8	8
Mean (SD)	115.3 (16.82)	40.3 (15.20)	105.5 (18.08)	29.8 (9.84)	110.4 (16.99)	35.0 (13.11)
Median	121.0	45.5	110.0	30.5	115.0	36.0
Min, Max	91, 128	18, 52	80, 122	17, 41	80, 128	17, 52
Week 104						
n	0	0	2	2	2	2
Mean (SD)			109.5 (7.78)	25.5 (9.19)	109.5 (7.78)	25.5 (9.19)
Median			109.5	25.5	109.5	25.5
Min, Max			104, 115	19, 32	104, 115	19, 32

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean (SD)	76.4 (8.55)		82.7 (7.53)		78.1 (8.60)	
Median	76.0		81.5		77.0	
Min, Max	60, 91		74, 92		60, 92	
Week 1						
n	15	15	5	5		
Mean (SD)	103.4 (9.13)	27.0 (9.67)	91.6 (10.78)	7.6 (3.85)		
Median	104.0	23.0	94.0	9.0		
Min, Max	87, 120	16, 45	78, 103	3, 11		
Week 2						
n	14	14	5	5		
Mean (SD)	108.6 (11.13)	32.1 (11.71)	92.2 (11.65)	9.0 (4.24)		
Median	111.0	28.0	95.0	8.0		
Min, Max	87, 126	19, 54	77, 106	3, 14		

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	14	14	4	4		
Mean (SD)	107.4 (11.32)	30.9 (10.02)	86.8 (9.71)	5.8 (3.20)		
Median	105.0	28.5	87.0	7.0		
Min, Max	91, 125	16, 55	75, 98	1, 8		
Week 4						
n	15	15	5	5		
Mean (SD)	108.1 (14.39)	31.7 (12.23)	85.6 (10.57)	2.4 (8.29)		
Median	111.0	32.0	89.0	1.0		
Min, Max	77, 131	5, 56	67, 93	-7, 15		
Week 6						
n	15	15	5	5		
Mean (SD)	106.1 (14.29)	30.6 (13.49)	89.4 (6.50)	6.2 (9.63)		
Median	106.0	32.0	90.0	5.0		
Min, Max	81, 127	5, 59	81, 99	-5, 16		

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Table GERMANY.2.6.2.3

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	15	15	6	6		
Mean (SD)	102.0 (18.60)	26.5 (20.01)	88.0 (7.43)	5.3 (6.02)		
Median	101.0	26.0	90.5	2.5		
Min, Max	64, 128	-14, 68	77, 96	0, 13		
Week 10						
n	10	10	6	6		
Mean (SD)	103.8 (12.59)	28.3 (13.32)	87.2 (10.72)	4.5 (7.01)		
Median	98.0	25.0	86.5	3.5		
Min, Max	90, 121	12, 54	73, 106	-4, 15		
Week 12						
n	12	12	6	6		
Mean (SD)	102.2 (15.64)	27.7 (17.04)	87.2 (11.46)	4.5 (6.25)		
Median	106.5	27.5	89.5	6.5		
Min, Max	68, 119	-8, 53	69, 99	-5, 11		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.3

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	10	10	5	5		
Mean (SD)	104.7 (11.91)	28.4 (10.33)	99.2 (9.86)	18.4 (6.35)		
Median	103.5	31.0	100.0	17.0		
Min, Max	88, 120	12, 45	86, 112	12, 28		
Week 14						
n	13	13	6	6		
Mean (SD)	103.2 (16.50)	26.6 (14.89)	116.7 (19.29)	34.0 (19.95)		
Median	103.0	32.0	113.5	28.0		
Min, Max	70, 125	-5, 46	93, 152	19, 72		
Week 15						
n	12	12	5	5		
Mean (SD)	100.5 (14.07)	24.6 (15.79)	112.8 (11.26)	30.2 (12.17)		
Median	100.0	26.5	110.0	30.0		
Min, Max	78, 125	-6, 50	97, 126	18, 50		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

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Table GERMANY.2.6.2.3

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	13	13	6	6		
Mean (SD)	101.5 (14.25)	25.0 (13.07)	112.0 (7.38)	29.3 (8.80)		
Median	105.0	28.0	110.0	29.0		
Min, Max	76, 122	0, 50	104, 122	18, 44		
Week 18						
n	11	11	6	6		
Mean (SD)	101.2 (16.06)	26.6 (15.66)	110.7 (9.03)	28.0 (11.80)		
Median	103.0	26.0	109.5	31.0		
Min, Max	75, 122	-1, 50	100, 124	11, 42		
Week 20						
n	10	10	5	5		
Mean (SD)	107.4 (16.11)	30.2 (16.14)	108.0 (12.49)	24.0 (17.59)		
Median	111.0	28.5	109.0	26.0		
Min, Max	73, 127	-3, 54	94, 123	2, 49		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.3
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	12	12	4	4		
Mean (SD)	105.5 (18.11)	29.6 (17.51)	110.3 (12.82)	28.3 (14.71)		
Median	110.0	34.5	114.0	28.5		
Min, Max	74, 128	-2, 53	93, 120	10, 46		
Week 24						
n	13	13	4	4		
Mean (SD)	103.5 (16.95)	26.9 (18.54)	109.3 (19.47)	27.3 (21.06)		
Median	100.0	30.0	114.5	29.0		
Min, Max	70, 130	-6, 58	83, 125	0, 51		
Week 28						
n	11	11	4	4	15	15
Mean (SD)	103.0 (19.29)	28.1 (21.02)	102.3 (21.85)	20.3 (22.51)	102.8 (19.19)	26.0 (20.91)
Median	103.0	32.0	109.0	26.5	104.0	29.0
Min, Max	60, 131	-16, 56	71, 120	-12, 40	60, 131	-16, 56

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.3

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	12	12	4	4	16	16
Mean (SD)	106.8 (16.88)	29.7 (18.18)	106.5 (18.48)	21.5 (21.27)	106.7 (16.65)	27.6 (18.61)
Median	111.0	30.0	111.5	20.0	111.0	27.5
Min, Max	75, 130	-1, 58	81, 122	-2, 48	75, 130	-2, 58
Week 36						
n	13	13	3	3	16	16
Mean (SD)	105.2 (17.22)	28.8 (18.43)	112.3 (16.74)	29.7 (19.04)	106.5 (16.82)	28.9 (17.89)
Median	110.0	30.0	122.0	31.0	112.0	30.5
Min, Max	73, 130	-3, 58	93, 122	10, 48	73, 130	-3, 58
Week 40						
n	11	11	4	4	15	15
Mean (SD)	105.0 (14.86)	29.7 (15.89)	109.8 (13.25)	28.8 (13.38)	106.3 (14.14)	29.5 (14.80)
Median	105.0	30.0	110.0	28.5	105.0	30.0
Min, Max	81, 137	6, 65	96, 123	13, 45	81, 137	6, 65

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.3

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	12	12	2	2	14	14
Mean (SD)	107.6 (19.76)	31.2 (18.63)	125.0 (5.66)	42.5 (17.68)	110.1 (19.31)	32.8 (18.30)
Median	109.0	29.0	125.0	42.5	111.0	30.0
Min, Max	73, 145	-3, 73	121, 129	30, 55	73, 145	-3, 73
Week 48						
n	9	9	3	3	12	12
Mean (SD)	111.1 (16.24)	35.0 (19.24)	119.0 (4.58)	38.7 (10.02)	113.1 (14.43)	35.9 (17.04)
Median	109.0	36.0	120.0	38.0	115.0	37.0
Min, Max	86, 140	10, 68	114, 123	29, 49	86, 140	10, 68
Week 52						
n	9	9	2	2	11	11
Mean (SD)	107.3 (12.93)	29.3 (12.30)	116.5 (14.85)	33.5 (27.58)	109.0 (13.02)	30.1 (14.14)
Median	107.0	29.0	116.5	33.5	107.0	29.0
Min, Max	85, 130	9, 44	106, 127	14, 53	85, 130	9, 53

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.3

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	9	9	3	3	12	12
Mean (SD)	108.0 (12.33)	31.6 (11.96)	114.7 (14.05)	34.0 (23.58)	109.7 (12.47)	32.2 (14.36)
Median	107.0	30.0	116.0	40.0	107.5	30.5
Min, Max	87, 124	12, 52	100, 128	8, 54	87, 128	8, 54
Week 60						
n	9	9	3	3	12	12
Mean (SD)	108.1 (16.59)	31.4 (17.50)	110.7 (14.57)	30.0 (23.64)	108.8 (15.49)	31.1 (18.02)
Median	102.0	32.0	109.0	33.0	105.5	32.0
Min, Max	92, 137	7, 65	97, 126	5, 52	92, 137	5, 65
Week 64						
n	6	6	3	3	9	9
Mean (SD)	111.7 (12.96)	35.2 (12.89)	114.0 (16.09)	33.3 (25.11)	112.4 (13.08)	34.6 (16.19)
Median	111.0	33.5	112.0	36.0	112.0	35.0
Min, Max	97, 131	21, 56	99, 131	7, 57	97, 131	7, 57

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.3

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	6	6	2	2	8	8
Mean (SD)	105.0 (11.01)	28.8 (9.68)	118.0 (19.80)	34.0 (31.11)	108.3 (13.37)	30.1 (14.53)
Median	107.0	28.5	118.0	34.0	107.0	28.5
Min, Max	90, 118	14, 42	104, 132	12, 56	90, 132	12, 56
Week 72						
n	4	4	0	0	4	4
Mean (SD)	94.5 (9.33)	22.5 (9.68)			94.5 (9.33)	22.5 (9.68)
Median	94.0	27.0			94.0	27.0
Min, Max	84, 106	8, 28			84, 106	8, 28
Week 80						
n	3	3	0	0	3	3
Mean (SD)	87.7 (3.21)	17.7 (9.07)			87.7 (3.21)	17.7 (9.07)
Median	89.0	19.0			89.0	19.0
Min, Max	84, 90	8, 26			84, 90	8, 26

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.3

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	4	4	1	1	5	5
Mean (SD)	104.8 (15.37)	26.5 (8.70)	110.0 (NA)	34.0 (NA)	105.8 (13.52)	28.0 (8.25)
Median	104.0	26.0	110.0	34.0	110.0	33.0
Min, Max	89, 122	19, 35	110, 110	34, 34	89, 122	19, 35
Week 96						
n	1	1	0	0	1	1
Mean (SD)	101.0 (NA)	23.0 (NA)			101.0 (NA)	23.0 (NA)
Median	101.0	23.0			101.0	23.0
Min, Max	101, 101	23, 23			101, 101	23, 23
Week 104						
n	1	1	0	0	1	1
Mean (SD)	109.0 (NA)	31.0 (NA)			109.0 (NA)	31.0 (NA)
Median	109.0	31.0			109.0	31.0
Min, Max	109, 109	31, 31			109, 109	31, 31

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.4
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	25		13		38	
Mean (SD)	80.5 (7.74)		79.2 (9.24)		80.1 (8.18)	
Median	81.0		81.0		81.0	
Min, Max	61, 94		61, 93		61, 94	
Week 1						
n	23	23	11	11		
Mean (SD)	102.5 (10.83)	20.9 (11.19)	91.7 (18.45)	12.5 (12.64)		
Median	103.0	22.0	93.0	11.0		
Min, Max	83, 122	-2, 43	62, 121	-3, 36		
Week 2						
n	25	25	12	12		
Mean (SD)	106.7 (13.08)	26.2 (13.45)	86.3 (15.13)	7.4 (10.04)		
Median	106.0	28.0	85.0	7.5		
Min, Max	84, 133	3, 54	64, 111	-9, 27		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.4
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	20	20	12	12		
Mean (SD)	106.5 (13.71)	24.8 (12.23)	91.4 (16.93)	12.5 (9.06)		
Median	105.0	28.0	92.5	11.5		
Min, Max	80, 125	-1, 40	58, 117	-3, 31		
Week 4						
n	25	25	13	13		
Mean (SD)	109.4 (17.30)	28.9 (15.00)	88.7 (16.55)	9.5 (9.66)		
Median	110.0	30.0	91.0	9.0		
Min, Max	74, 139	-7, 58	58, 114	-5, 30		
Week 6						
n	21	21	13	13		
Mean (SD)	107.9 (13.30)	27.9 (11.57)	88.2 (11.36)	9.0 (6.30)		
Median	108.0	26.0	88.0	8.0		
Min, Max	85, 127	8, 47	62, 107	-2, 19		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.6.2.4
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	21	21	12	12		
Mean (SD)	102.6 (18.14)	22.8 (16.24)	88.8 (15.15)	9.3 (10.02)		
Median	101.0	20.0	89.0	11.0		
Min, Max	64, 130	-14, 48	67, 112	-9, 26		
Week 10						
n	17	17	11	11		
Mean (SD)	107.2 (14.01)	25.4 (11.40)	88.2 (12.57)	8.5 (8.82)		
Median	106.0	23.0	88.0	11.0		
Min, Max	81, 130	9, 47	62, 105	-7, 20		
Week 12						
n	19	19	12	12		
Mean (SD)	104.8 (13.40)	25.3 (10.35)	84.9 (12.93)	4.3 (7.43)		
Median	105.0	25.0	86.5	2.5		
Min, Max	77, 123	9, 42	54, 106	-7, 21		

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Table GERMANY.2.6.2.4
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	18	18	12	12		
Mean (SD)	106.9 (13.69)	25.8 (11.56)	97.1 (9.86)	19.0 (8.26)		
Median	105.0	24.0	99.0	19.5		
Min, Max	87, 129	4, 48	76, 110	1, 32		
Week 14						
n	18	18	12	12		
Mean (SD)	106.8 (15.25)	26.7 (14.67)	105.8 (14.58)	26.3 (12.23)		
Median	106.5	31.0	105.0	26.5		
Min, Max	70, 129	-5, 45	89, 137	4, 45		
Week 15						
n	16	16	11	11		
Mean (SD)	100.8 (12.33)	21.6 (13.00)	108.5 (15.79)	28.2 (13.01)		
Median	101.0	19.5	105.0	26.0		
Min, Max	80, 124	-6, 41	87, 141	7, 50		

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Table GERMANY.2.6.2.4
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	22	22	13	13		
Mean (SD)	106.4 (15.39)	25.9 (15.54)	104.3 (18.26)	25.1 (15.68)		
Median	103.5	27.0	95.0	27.0		
Min, Max	78, 137	2, 53	87, 153	5, 60		
Week 18						
n	18	18	11	11		
Mean (SD)	98.4 (15.27)	19.5 (16.75)	103.8 (16.66)	23.7 (13.01)		
Median	98.5	13.5	98.0	23.0		
Min, Max	78, 122	-4, 50	76, 139	0, 46		
Week 20						
n	16	16	10	10		
Mean (SD)	104.0 (16.07)	22.5 (15.05)	102.7 (13.60)	21.2 (14.26)		
Median	107.5	25.5	99.5	16.5		
Min, Max	76, 129	2, 48	84, 136	8, 48		

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Table GERMANY.2.6.2.4
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	21	21	10	10		
Mean (SD)	107.7 (16.90)	27.5 (18.11)	107.6 (16.91)	26.8 (15.89)		
Median	109.0	22.0	107.0	27.0		
Min, Max	84, 132	4, 67	81, 143	5, 50		
Week 24						
n	22	22	10	10		
Mean (SD)	102.3 (15.14)	22.2 (15.30)	104.0 (16.15)	23.5 (14.68)		
Median	102.0	19.5	104.0	21.0		
Min, Max	77, 130	-4, 58	79, 136	3, 44		
Week 28						
n	18	18	10	10	28	28
Mean (SD)	103.1 (17.36)	25.1 (15.37)	105.7 (12.57)	24.4 (13.83)	104.0 (15.62)	24.9 (14.58)
Median	99.5	20.0	101.5	19.0	101.0	20.0
Min, Max	67, 142	0, 58	93, 131	7, 50	67, 142	0, 58

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Table GERMANY.2.6.2.4
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	18	18	10	10	28	28
Mean (SD)	104.0 (17.53)	23.9 (16.60)	101.0 (9.27)	21.2 (12.07)	102.9 (14.98)	23.0 (14.96)
Median	104.0	23.0	100.0	19.0	101.5	23.0
Min, Max	66, 132	-9, 58	86, 118	8, 45	66, 132	-9, 58
Week 36						
n	14	14	12	12	26	26
Mean (SD)	107.9 (16.89)	29.6 (16.83)	105.8 (13.63)	26.3 (17.24)	106.9 (15.21)	28.0 (16.76)
Median	112.0	31.0	105.0	19.0	108.0	26.0
Min, Max	82, 130	-1, 58	86, 124	8, 61	82, 130	-1, 61
Week 40						
n	14	14	11	11	25	25
Mean (SD)	101.4 (17.95)	23.5 (18.73)	104.1 (12.23)	23.3 (13.93)	102.6 (15.45)	23.4 (16.46)
Median	101.0	21.0	98.0	22.0	99.0	21.0
Min, Max	76, 137	-1, 65	89, 127	7, 53	76, 137	-1, 65

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Table GERMANY.2.6.2.4
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	16	16	10	10	26	26
Mean (SD)	107.5 (22.65)	28.6 (21.70)	107.0 (12.51)	27.8 (14.44)	107.3 (19.08)	28.3 (18.92)
Median	110.0	29.0	105.0	26.5	107.0	29.0
Min, Max	58, 145	-17, 73	89, 125	11, 53	58, 145	-17, 73
Week 48						
n	12	12	12	12	24	24
Mean (SD)	102.8 (17.98)	23.5 (19.29)	106.3 (12.51)	26.8 (14.20)	104.5 (15.25)	25.1 (16.65)
Median	100.0	16.0	111.0	31.0	103.5	24.0
Min, Max	79, 140	6, 68	89, 129	4, 50	79, 140	4, 68
Week 52						
n	11	11	10	10	21	21
Mean (SD)	108.2 (17.99)	29.0 (14.06)	108.6 (16.85)	28.6 (14.33)	108.4 (17.02)	28.8 (13.83)
Median	108.0	30.0	107.5	30.5	108.0	30.0
Min, Max	85, 136	9, 48	86, 142	5, 49	85, 142	5, 49

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Table GERMANY.2.6.2.4
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
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Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	11	11	10	10	21	21
Mean (SD)	105.9 (14.62)	27.7 (15.47)	104.7 (15.30)	26.4 (14.79)	105.3 (14.58)	27.1 (14.78)
Median	105.0	30.0	109.0	26.0	107.0	29.0
Min, Max	88, 126	2, 52	78, 124	-3, 46	78, 126	-3, 52
Week 60						
n	10	10	9	9	19	19
Mean (SD)	109.6 (16.42)	31.4 (15.96)	100.6 (19.01)	19.2 (14.16)	105.3 (17.80)	25.6 (15.98)
Median	113.5	32.5	103.0	14.0	105.0	22.0
Min, Max	89, 137	7, 65	75, 141	4, 48	75, 141	4, 65
Week 64						
n	6	6	8	8	14	14
Mean (SD)	102.5 (15.15)	27.2 (9.70)	97.9 (16.47)	18.8 (13.38)	99.9 (15.49)	22.4 (12.30)
Median	103.5	23.0	100.5	17.0	101.0	21.5
Min, Max	79, 121	18, 43	72, 116	-2, 36	72, 121	-2, 43

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Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	7	7	7	7	14	14
Mean (SD)	103.4 (19.36)	26.9 (13.03)	99.7 (22.66)	20.1 (20.41)	101.6 (20.34)	23.5 (16.81)
Median	114.0	29.0	107.0	22.0	107.0	23.0
Min, Max	74, 124	13, 43	66, 132	-3, 56	66, 132	-3, 56
Week 72						
n	6	6	6	6	12	12
Mean (SD)	109.7 (18.42)	30.7 (14.17)	93.5 (18.83)	13.3 (18.01)	101.6 (19.67)	22.0 (17.90)
Median	110.0	32.0	88.0	13.0	101.0	23.5
Min, Max	84, 135	8, 47	70, 122	-11, 39	70, 135	-11, 47
Week 80						
n	3	3	4	4	7	7
Mean (SD)	102.7 (25.72)	26.0 (22.34)	102.8 (23.96)	25.3 (14.38)	102.7 (22.53)	25.6 (16.43)
Median	92.0	19.0	113.0	31.0	112.0	29.0
Min, Max	84, 132	8, 51	67, 118	4, 35	67, 132	4, 51

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	5	5	11	11
Mean (SD)	107.3 (15.00)	26.5 (10.15)	98.6 (14.42)	21.0 (9.41)	103.4 (14.71)	24.0 (9.76)
Median	114.0	30.0	104.0	19.0	110.0	27.0
Min, Max	83, 122	10, 35	76, 110	12, 34	76, 122	10, 35
Week 96						
n	3	3	3	3	6	6
Mean (SD)	106.7 (19.14)	29.3 (15.50)	105.0 (22.11)	29.3 (12.01)	105.8 (18.52)	29.3 (12.40)
Median	101.0	23.0	113.0	30.0	107.0	26.5
Min, Max	91, 128	18, 47	80, 122	17, 41	80, 128	17, 47
Week 104						
n	1	1	2	2	3	3
Mean (SD)	109.0 (NA)	31.0 (NA)	109.5 (7.78)	25.5 (9.19)	109.3 (5.51)	27.3 (7.23)
Median	109.0	31.0	109.5	25.5	109.0	31.0
Min, Max	109, 109	31, 31	104, 115	19, 32	104, 115	19, 32

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Table GERMANY.2.6.2.4
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
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Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		13		45	
Mean (SD)	73.8 (9.76)		78.7 (11.86)		75.2 (10.51)	
Median	75.0		80.0		76.0	
Min, Max	55, 91		54, 93		54, 93	
Week 1						
n	27	27	12	12		
Mean (SD)	95.3 (11.65)	21.8 (9.66)	84.8 (12.17)	7.3 (6.89)		
Median	96.0	19.0	83.5	8.5		
Min, Max	70, 116	2, 45	62, 103	-5, 17		
Week 2						
n	30	30	11	11		
Mean (SD)	102.9 (10.80)	29.2 (11.35)	89.3 (11.71)	12.0 (8.15)		
Median	104.0	25.5	86.0	12.0		
Min, Max	80, 122	15, 55	74, 109	3, 32		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.4
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	26	26	10	10		
Mean (SD)	104.8 (12.92)	32.2 (13.48)	83.5 (12.63)	7.7 (10.93)		
Median	104.5	30.5	83.0	6.5		
Min, Max	82, 126	13, 59	64, 105	-3, 34		
Week 4						
n	27	27	11	11		
Mean (SD)	106.1 (14.70)	32.2 (12.17)	84.4 (10.84)	7.1 (10.04)		
Median	108.0	32.0	87.0	6.0		
Min, Max	74, 126	10, 56	67, 103	-7, 25		
Week 6						
n	29	29	11	11		
Mean (SD)	106.0 (16.02)	31.5 (14.11)	84.5 (10.41)	7.3 (7.99)		
Median	107.0	32.0	87.0	8.0		
Min, Max	73, 133	2, 59	70, 99	-5, 19		

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.
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and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	25	25	12	12		
Mean (SD)	104.6 (14.00)	30.8 (15.19)	85.5 (9.62)	8.0 (6.67)		
Median	104.0	32.0	86.0	9.0		
Min, Max	80, 128	4, 68	66, 99	0, 21		
Week 10						
n	21	21	11	11		
Mean (SD)	106.5 (14.55)	32.6 (14.27)	81.5 (15.49)	1.8 (9.01)		
Median	112.0	33.0	83.0	3.0		
Min, Max	80, 128	9, 54	51, 106	-20, 15		
Week 12						
n	28	28	12	12		
Mean (SD)	106.6 (16.30)	33.3 (16.76)	87.4 (9.03)	9.9 (9.13)		
Median	113.0	32.5	88.0	9.5		
Min, Max	68, 131	-8, 60	69, 99	-5, 31		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.
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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Table GERMANY.2.6.2.4
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and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	24	24	9	9		
Mean (SD)	106.0 (17.49)	32.6 (16.47)	96.0 (11.79)	17.1 (7.82)		
Median	108.0	32.0	94.0	16.0		
Min, Max	70, 135	-1, 59	75, 112	4, 31		
Week 14						
n	27	27	10	10		
Mean (SD)	106.4 (19.80)	32.3 (18.30)	108.2 (19.49)	28.0 (16.51)		
Median	110.0	32.0	110.5	25.5		
Min, Max	70, 146	3, 69	81, 152	10, 72		
Week 15						
n	24	24	8	8		
Mean (SD)	108.8 (17.55)	33.4 (16.30)	105.0 (12.68)	26.3 (7.13)		
Median	113.0	32.0	109.5	29.5		
Min, Max	75, 145	2, 64	85, 121	14, 33		

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Table GERMANY.2.6.2.4
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and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	24	24	11	11		
Mean (SD)	105.8 (19.22)	31.0 (16.66)	106.3 (14.79)	28.5 (13.72)		
Median	108.0	29.0	110.0	29.0		
Min, Max	67, 147	-4, 66	74, 122	3, 60		
Week 18						
n	22	22	9	9		
Mean (SD)	103.6 (20.45)	29.4 (19.13)	102.1 (17.91)	22.9 (12.57)		
Median	104.5	27.0	106.0	29.0		
Min, Max	69, 143	-4, 63	73, 124	2, 36		
Week 20						
n	25	25	9	9		
Mean (SD)	106.3 (19.81)	31.9 (17.63)	100.9 (20.81)	21.7 (17.69)		
Median	109.0	32.0	104.0	26.0		
Min, Max	67, 145	-4, 64	66, 125	-5, 49		

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and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	22	22	9	9		
Mean (SD)	106.7 (21.88)	33.3 (20.71)	103.9 (17.29)	28.9 (17.06)		
Median	113.0	37.0	107.0	29.0		
Min, Max	65, 146	-6, 66	70, 125	-1, 53		
Week 24						
n	25	25	8	8		
Mean (SD)	109.1 (20.26)	34.9 (19.50)	104.9 (19.98)	29.4 (15.34)		
Median	111.0	37.0	105.5	29.5		
Min, Max	70, 152	-6, 71	76, 129	0, 51		
Week 28						
n	24	24	8	8	32	32
Mean (SD)	106.3 (20.96)	31.5 (20.72)	99.3 (22.76)	23.8 (19.86)	104.5 (21.27)	29.6 (20.48)
Median	106.0	31.0	101.0	26.5	105.0	29.5
Min, Max	60, 148	-16, 67	64, 129	-12, 45	60, 148	-16, 67

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Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	26	26	8	8	34	34
Mean (SD)	105.1 (18.21)	30.9 (19.96)	101.6 (22.10)	24.6 (17.58)	104.3 (18.90)	29.4 (19.35)
Median	106.5	30.0	100.5	23.5	105.0	29.0
Min, Max	68, 133	-8, 71	66, 132	-2, 48	66, 133	-8, 71
Week 36						
n	21	21	8	8	29	29
Mean (SD)	105.9 (18.30)	32.2 (17.44)	110.8 (12.89)	34.1 (12.41)	107.2 (16.91)	32.7 (16.02)
Median	109.0	32.0	114.0	32.0	109.0	32.0
Min, Max	73, 143	-3, 62	93, 123	10, 50	73, 143	-3, 62
Week 40						
n	23	23	7	7	30	30
Mean (SD)	109.4 (18.84)	35.5 (19.15)	103.0 (24.85)	28.1 (18.73)	107.9 (20.12)	33.8 (18.99)
Median	108.0	33.0	102.0	32.0	107.5	33.0
Min, Max	66, 155	-1, 74	57, 131	-5, 47	57, 155	-5, 74

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Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	21	21	6	6	27	27
Mean (SD)	105.8 (17.97)	31.5 (15.61)	112.8 (15.99)	39.3 (10.84)	107.4 (17.51)	33.2 (14.87)
Median	107.0	30.0	111.5	39.0	107.0	34.0
Min, Max	65, 146	4, 65	96, 131	26, 55	65, 146	4, 65
Week 48						
n	19	19	6	6	25	25
Mean (SD)	107.4 (19.10)	33.0 (17.17)	107.5 (19.66)	34.0 (10.39)	107.4 (18.82)	33.2 (15.61)
Median	109.0	34.0	114.0	31.5	109.0	34.0
Min, Max	68, 153	3, 72	82, 127	20, 49	68, 153	3, 72
Week 52						
n	16	16	6	6	22	22
Mean (SD)	106.9 (20.28)	33.1 (17.03)	101.3 (24.74)	27.7 (18.26)	105.4 (21.12)	31.6 (17.11)
Median	108.5	37.0	105.5	28.0	107.5	34.0
Min, Max	62, 143	-3, 62	64, 127	2, 53	62, 143	-3, 62

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Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	21	21	6	6	27	27
Mean (SD)	105.1 (23.64)	29.8 (19.28)	105.2 (18.19)	31.5 (15.42)	105.1 (22.21)	30.2 (18.23)
Median	109.0	33.0	101.5	29.5	107.0	32.0
Min, Max	52, 148	-5, 67	81, 128	8, 54	52, 148	-5, 67
Week 60						
n	14	14	6	6	20	20
Mean (SD)	103.9 (22.70)	29.3 (19.74)	103.5 (17.47)	29.8 (15.87)	103.8 (20.81)	29.5 (18.25)
Median	102.0	32.0	98.0	29.5	100.5	31.0
Min, Max	60, 151	-2, 70	84, 126	5, 52	60, 151	-2, 70
Week 64						
n	15	15	6	6	21	21
Mean (SD)	105.3 (20.77)	30.6 (17.02)	108.2 (17.27)	34.5 (16.23)	106.1 (19.45)	31.7 (16.49)
Median	106.0	28.0	106.0	37.0	106.0	32.0
Min, Max	47, 137	-10, 56	84, 131	7, 57	47, 137	-10, 57

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	12	12	5	5	17	17
Mean (SD)	105.3 (20.28)	31.0 (17.26)	101.0 (10.63)	27.4 (9.69)	104.0 (17.75)	29.9 (15.20)
Median	108.5	33.0	104.0	29.0	108.0	32.0
Min, Max	50, 129	-7, 59	88, 113	12, 38	50, 129	-7, 59
Week 72						
n	10	10	3	3	13	13
Mean (SD)	101.8 (15.30)	29.4 (11.67)	108.0 (16.52)	34.0 (5.57)	103.2 (15.12)	30.5 (10.56)
Median	106.0	27.0	109.0	33.0	107.0	29.0
Min, Max	68, 119	11, 46	91, 124	29, 40	68, 124	11, 46
Week 80						
n	8	8	3	3	11	11
Mean (SD)	102.4 (15.24)	26.3 (16.42)	107.3 (14.64)	33.3 (5.13)	103.7 (14.52)	28.2 (14.32)
Median	100.0	22.5	105.0	32.0	104.0	29.0
Min, Max	87, 132	8, 59	94, 123	29, 39	87, 132	8, 59

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Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	3	3	1	1	4	4
Mean (SD)	96.3 (28.71)	28.0 (23.81)	98.0 (NA)	22.0 (NA)	96.8 (23.46)	26.5 (19.67)
Median	89.0	19.0	98.0	22.0	93.5	20.5
Min, Max	72, 128	10, 55	98, 98	22, 22	72, 128	10, 55
Week 96						
n	2	2	1	1	3	3
Mean (SD)	121.0 (5.66)	48.0 (5.66)	107.0 (NA)	31.0 (NA)	116.3 (9.02)	42.3 (10.60)
Median	121.0	48.0	107.0	31.0	117.0	44.0
Min, Max	117, 125	44, 52	107, 107	31, 31	107, 125	31, 52

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.5

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by the Randomization Stratification Factors of Transfusion History
(> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		10		34	
Mean (SD)	74.3 (8.64)		78.8 (8.34)		75.6 (8.68)	
Median	76.0		79.5		76.5	
Min, Max	57, 88		62, 92		57, 92	
Week 1						
n	20	20	10	10		
Mean (SD)	96.7 (12.66)	22.7 (9.82)	92.1 (15.90)	13.3 (12.87)		
Median	92.5	21.0	87.0	10.5		
Min, Max	70, 120	9, 45	78, 121	-3, 36		
Week 2						
n	23	23	10	10		
Mean (SD)	102.1 (12.22)	27.9 (11.03)	88.3 (13.86)	9.5 (10.49)		
Median	104.0	26.0	87.5	12.0		
Min, Max	80, 126	4, 55	70, 106	-9, 27		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.5

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	18	18	9	9		
Mean (SD)	103.4 (13.65)	30.0 (13.47)	85.7 (11.91)	8.3 (6.71)		
Median	104.0	28.5	90.0	7.0		
Min, Max	83, 125	1, 59	64, 98	-3, 20		
Week 4						
n	21	21	10	10		
Mean (SD)	106.3 (14.98)	31.8 (12.16)	88.0 (8.73)	9.2 (10.03)		
Median	106.0	32.0	87.5	5.5		
Min, Max	81, 139	12, 58	77, 108	1, 30		
Week 6						
n	21	21	10	10		
Mean (SD)	104.6 (17.24)	29.9 (14.96)	87.3 (10.53)	8.5 (7.37)		
Median	104.0	28.0	87.5	8.0		
Min, Max	73, 133	2, 59	70, 101	-5, 19		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1. Change from baseline is summarized only for patients who have data at baseline and the specified time point. Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.6.2.5

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	18	18	9	9		
Mean (SD)	96.8 (16.28)	23.9 (18.47)	89.1 (11.67)	10.0 (10.91)		
Median	96.5	20.0	89.0	10.0		
Min, Max	64, 128	-14, 68	72, 112	-9, 26		
Week 10						
n	14	14	8	8		
Mean (SD)	101.3 (14.78)	26.9 (10.96)	84.1 (18.04)	4.9 (12.61)		
Median	98.0	28.0	88.0	8.0		
Min, Max	80, 122	9, 47	51, 105	-20, 19		
Week 12						
n	22	22	10	10		
Mean (SD)	102.6 (16.92)	28.3 (15.52)	85.6 (6.80)	6.8 (6.25)		
Median	104.5	27.5	87.0	5.0		
Min, Max	68, 131	-8, 58	76, 97	0, 18		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1. Change from baseline is summarized only for patients who have data at baseline and the specified time point. Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.5

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by the Randomization Stratification Factors of Transfusion History
(> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	17	17	9	9		
Mean (SD)	102.3 (17.71)	28.8 (15.15)	95.2 (10.52)	17.9 (10.71)		
Median	99.0	27.0	99.0	18.0		
Min, Max	70, 135	-1, 58	75, 110	1, 32		
Week 14						
n	19	19	9	9		
Mean (SD)	105.4 (18.68)	30.4 (17.55)	101.1 (13.70)	22.0 (12.00)		
Median	103.0	29.0	102.0	21.0		
Min, Max	74, 146	3, 69	81, 123	4, 45		
Week 15						
n	17	17	9	9		
Mean (SD)	100.2 (19.49)	25.1 (17.41)	100.9 (10.95)	22.6 (9.11)		
Median	95.0	27.0	100.0	24.0		
Min, Max	75, 145	-6, 64	85, 119	7, 36		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by the Randomization Stratification Factors of Transfusion History
(> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	18	18	10	10		
Mean (SD)	102.7 (22.62)	27.4 (19.12)	96.5 (13.95)	17.7 (10.51)		
Median	100.5	28.0	92.5	16.0		
Min, Max	67, 147	-4, 66	74, 119	3, 36		
Week 18						
n	18	18	9	9		
Mean (SD)	98.9 (20.98)	24.3 (18.09)	91.2 (13.49)	13.2 (8.26)		
Median	95.0	24.5	95.0	14.0		
Min, Max	69, 143	-4, 62	73, 109	0, 26		
Week 20						
n	16	16	9	9		
Mean (SD)	102.4 (24.26)	27.5 (20.15)	91.0 (13.85)	11.9 (10.03)		
Median	103.5	29.0	95.0	11.0		
Min, Max	67, 145	-4, 64	66, 106	-5, 28		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	18	18	9	9		
Mean (SD)	105.2 (23.30)	30.1 (19.61)	96.8 (15.51)	19.4 (13.97)		
Median	97.5	33.0	96.0	20.0		
Min, Max	65, 146	-6, 65	70, 121	-1, 38		
Week 24						
n	18	18	7	7		
Mean (SD)	104.9 (21.84)	29.4 (18.98)	95.0 (14.19)	18.0 (14.67)		
Median	98.5	30.5	92.0	16.0		
Min, Max	70, 152	-6, 71	79, 116	0, 35		
Week 28						
n	17	17	7	7	24	24
Mean (SD)	101.6 (23.92)	26.2 (20.19)	92.0 (19.75)	13.9 (16.93)	98.8 (22.80)	22.6 (19.79)
Median	98.0	22.0	96.0	16.0	96.5	20.0
Min, Max	60, 148	-16, 67	64, 124	-12, 41	60, 148	-16, 67

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Table GERMANY.2.6.2.5

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by the Randomization Stratification Factors of Transfusion History
(> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	19	19	9	9	28	28
Mean (SD)	100.7 (20.41)	26.4 (19.31)	97.6 (11.51)	17.9 (12.15)	99.7 (17.87)	23.7 (17.58)
Median	101.0	25.0	99.0	15.0	101.0	23.5
Min, Max	66, 133	-9, 71	81, 118	-2, 35	66, 133	-9, 71
Week 36						
n	13	13	8	8	21	21
Mean (SD)	101.2 (19.18)	26.8 (15.89)	96.6 (11.56)	18.5 (11.34)	99.5 (16.51)	23.6 (14.61)
Median	102.0	28.0	93.5	15.0	95.0	25.0
Min, Max	73, 143	-3, 62	86, 122	8, 39	73, 143	-3, 62
Week 40						
n	16	16	7	7	23	23
Mean (SD)	105.8 (23.97)	31.4 (22.12)	93.4 (19.20)	15.3 (13.20)	102.0 (22.93)	26.5 (20.94)
Median	104.0	31.5	96.0	13.0	98.0	22.0
Min, Max	66, 155	-1, 74	57, 122	-5, 39	57, 155	-5, 74

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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(> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	14	14	5	5	19	19
Mean (SD)	99.9 (25.08)	26.6 (22.84)	100.6 (11.55)	24.6 (11.24)	100.1 (22.00)	26.1 (20.14)
Median	102.5	27.0	98.0	21.0	98.0	24.0
Min, Max	58, 146	-17, 65	89, 120	13, 37	58, 146	-17, 65
Week 48						
n	13	13	6	6	19	19
Mean (SD)	103.9 (22.45)	27.5 (19.20)	96.2 (13.82)	18.5 (12.44)	101.5 (20.07)	24.7 (17.53)
Median	108.0	29.0	89.5	16.5	97.0	20.0
Min, Max	68, 153	6, 72	82, 116	4, 33	68, 153	4, 72
Week 52						
n	12	12	6	6	18	18
Mean (SD)	104.7 (23.75)	28.9 (17.33)	95.8 (20.58)	15.8 (15.47)	101.7 (22.53)	24.6 (17.47)
Median	107.5	24.5	97.0	10.0	104.0	22.0
Min, Max	62, 143	5, 62	64, 125	2, 42	62, 143	2, 62

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	10	10	6	6	16	16
Mean (SD)	102.5 (25.21)	28.7 (18.94)	99.8 (16.25)	19.8 (16.24)	101.5 (21.70)	25.4 (17.97)
Median	106.0	29.0	97.0	20.5	102.5	28.0
Min, Max	52, 148	-5, 67	78, 124	-3, 41	52, 148	-5, 67
Week 60						
n	10	10	6	6	16	16
Mean (SD)	102.8 (24.14)	27.7 (19.36)	93.0 (7.01)	13.0 (10.62)	99.1 (19.75)	22.2 (17.79)
Median	98.5	27.0	90.5	9.5	94.0	19.5
Min, Max	60, 151	3, 70	85, 105	4, 29	60, 151	3, 70
Week 64						
n	9	9	6	6	15	15
Mean (SD)	101.2 (24.91)	27.6 (17.97)	96.7 (12.23)	16.7 (15.59)	99.4 (20.33)	23.2 (17.37)
Median	101.0	28.0	97.5	12.0	99.0	24.0
Min, Max	47, 137	-10, 56	79, 116	-2, 38	47, 137	-10, 56

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	9	9	5	5	14	14
Mean (SD)	101.8 (24.47)	28.0 (19.93)	95.6 (14.72)	14.8 (14.65)	99.6 (21.09)	23.3 (18.80)
Median	108.0	28.0	92.0	12.0	99.5	24.0
Min, Max	50, 129	-7, 59	78, 116	-3, 33	50, 129	-7, 59
Week 72						
n	7	7	4	4	11	11
Mean (SD)	98.1 (20.84)	25.7 (14.87)	93.5 (21.42)	15.5 (22.71)	96.5 (20.10)	22.0 (17.72)
Median	96.0	23.0	91.0	17.0	93.0	23.0
Min, Max	68, 135	8, 47	70, 122	-11, 39	68, 135	-11, 47
Week 80						
n	2	2	3	3	5	5
Mean (SD)	88.0 (5.66)	13.5 (7.78)	106.7 (11.02)	31.3 (2.08)	99.2 (13.16)	24.2 (10.62)
Median	88.0	13.5	112.0	32.0	94.0	29.0
Min, Max	84, 92	8, 19	94, 114	29, 33	84, 114	8, 33

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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ALXN2040-PNH-301

Table GERMANY.2.6.2.5

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	5	5	2	2	7	7
Mean (SD)	95.6 (18.68)	20.2 (10.89)	101.5 (12.02)	19.5 (10.61)	97.3 (16.28)	20.0 (9.90)
Median	95.0	19.0	101.5	19.5	95.0	19.0
Min, Max	72, 115	10, 35	93, 110	12, 27	72, 115	10, 35
Week 96						
n	3	3	2	2	5	5
Mean (SD)	103.0 (13.11)	28.3 (13.80)	117.5 (6.36)	35.5 (7.78)	108.8 (12.62)	31.2 (11.21)
Median	101.0	23.0	117.5	35.5	113.0	30.0
Min, Max	91, 117	18, 44	113, 122	30, 41	91, 122	18, 44
Week 104						
n	1	1	1	1	2	2
Mean (SD)	109.0 (NA)	31.0 (NA)	115.0 (NA)	32.0 (NA)	112.0 (4.24)	31.5 (0.71)
Median	109.0	31.0	115.0	32.0	112.0	31.5
Min, Max	109, 109	31, 31	115, 115	32, 32	109, 115	31, 32

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		16		49	
Mean (SD)	78.5 (9.79)		79.1 (11.80)		78.7 (10.37)	
Median	79.0		81.5		80.0	
Min, Max	55, 94		54, 93		54, 94	
Week 1						
n	30	30	13	13		
Mean (SD)	99.9 (11.09)	20.5 (10.67)	85.0 (15.14)	7.1 (6.90)		
Median	100.5	19.5	84.0	9.0		
Min, Max	75, 122	-2, 43	62, 108	-5, 15		
Week 2						
n	32	32	13	13		
Mean (SD)	106.4 (11.60)	27.8 (13.35)	87.3 (13.55)	9.7 (8.67)		
Median	108.5	26.0	86.0	7.0		
Min, Max	84, 133	3, 54	64, 111	1, 32		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	28	28	13	13		
Mean (SD)	106.9 (12.87)	28.3 (13.47)	89.3 (17.62)	11.7 (11.85)		
Median	106.0	27.5	84.0	8.0		
Min, Max	80, 126	-1, 58	58, 117	-3, 34		
Week 4						
n	31	31	14	14		
Mean (SD)	108.6 (16.72)	29.8 (14.59)	85.8 (17.23)	7.8 (9.78)		
Median	111.0	31.0	90.0	10.0		
Min, Max	74, 131	-7, 56	58, 114	-7, 21		
Week 6						
n	29	29	14	14		
Mean (SD)	108.4 (12.88)	30.0 (11.85)	86.0 (11.45)	8.0 (7.03)		
Median	108.0	29.0	87.0	7.5		
Min, Max	80, 127	9, 50	62, 107	-2, 19		

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by the Randomization Stratification Factors of Transfusion History
(> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	28	28	15	15		
Mean (SD)	108.0 (14.21)	29.3 (14.20)	85.9 (13.26)	7.8 (6.68)		
Median	108.5	31.5	89.0	9.0		
Min, Max	81, 130	5, 48	66, 106	-7, 17		
Week 10						
n	24	24	14	14		
Mean (SD)	110.0 (12.97)	30.8 (14.66)	85.2 (12.23)	5.4 (7.47)		
Median	111.5	28.5	83.0	4.5		
Min, Max	83, 130	9, 54	62, 106	-7, 20		
Week 12						
n	25	25	14	14		
Mean (SD)	108.8 (12.88)	31.6 (14.51)	86.6 (13.45)	7.4 (10.22)		
Median	114.0	31.0	87.0	7.5		
Min, Max	86, 125	3, 60	54, 106	-7, 31		

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by the Randomization Stratification Factors of Transfusion History
(> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	25	25	12	12		
Mean (SD)	109.2 (14.03)	30.3 (14.86)	97.7 (10.75)	18.4 (5.55)		
Median	115.0	31.0	100.5	16.0		
Min, Max	80, 130	4, 59	76, 112	12, 28		
Week 14						
n	26	26	13	13		
Mean (SD)	107.4 (17.69)	29.8 (16.92)	110.9 (17.74)	30.6 (14.62)		
Median	111.0	33.5	110.0	26.0		
Min, Max	70, 138	-5, 61	89, 152	13, 72		
Week 15						
n	23	23	10	10		
Mean (SD)	109.5 (11.76)	31.3 (14.73)	112.6 (15.17)	31.7 (10.56)		
Median	113.0	33.0	112.0	30.0		
Min, Max	88, 135	7, 62	87, 141	15, 50		

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by the Randomization Stratification Factors of Transfusion History
(> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	28	28	14	14		
Mean (SD)	108.2 (12.82)	29.3 (14.27)	111.4 (15.59)	33.0 (13.98)		
Median	108.5	30.0	109.0	31.0		
Min, Max	84, 137	2, 63	93, 153	12, 60		
Week 18						
n	22	22	11	11		
Mean (SD)	103.2 (15.97)	25.5 (19.33)	112.7 (12.70)	31.6 (8.58)		
Median	104.5	24.5	112.0	31.0		
Min, Max	73, 124	-4, 63	92, 139	15, 46		
Week 20						
n	25	25	10	10		
Mean (SD)	107.3 (13.36)	28.7 (15.30)	111.6 (13.33)	30.0 (14.82)		
Median	109.0	28.0	109.0	28.5		
Min, Max	78, 127	3, 54	95, 136	10, 49		

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by the Randomization Stratification Factors of Transfusion History
(> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	25	25	10	10		
Mean (SD)	108.6 (16.37)	30.8 (19.78)	114.0 (13.74)	35.3 (14.41)		
Median	110.0	32.0	111.0	35.0		
Min, Max	74, 131	4, 67	97, 143	8, 53		
Week 24						
n	29	29	11	11		
Mean (SD)	106.5 (15.90)	28.6 (18.71)	110.4 (17.11)	31.3 (13.02)		
Median	104.0	29.0	106.0	29.0		
Min, Max	70, 136	-4, 59	76, 136	12, 51		
Week 28						
n	25	25	11	11	36	36
Mean (SD)	107.2 (15.64)	30.5 (17.80)	109.7 (12.44)	30.6 (12.50)	107.9 (14.61)	30.5 (16.18)
Median	105.0	30.0	104.0	29.0	105.0	29.5
Min, Max	85, 142	3, 62	94, 131	13, 50	85, 142	3, 62

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	25	25	9	9	34	34
Mean (SD)	107.7 (15.14)	29.3 (18.65)	105.0 (18.99)	27.6 (15.52)	107.0 (15.99)	28.8 (17.66)
Median	108.0	29.0	106.0	23.0	107.0	27.0
Min, Max	82, 132	-8, 64	66, 132	10, 48	66, 132	-8, 64
Week 36						
n	22	22	12	12	34	34
Mean (SD)	109.9 (16.07)	33.7 (17.45)	115.2 (8.20)	36.7 (14.02)	111.7 (13.91)	34.8 (16.17)
Median	113.0	36.0	118.0	31.5	115.5	33.0
Min, Max	78, 130	-1, 58	102, 124	17, 61	78, 130	-1, 61
Week 40						
n	21	21	11	11	32	32
Mean (SD)	106.9 (14.02)	30.7 (18.08)	110.2 (13.40)	31.5 (14.08)	108.0 (13.69)	30.9 (16.59)
Median	106.0	33.0	109.0	32.0	106.5	32.5
Min, Max	78, 137	-1, 65	93, 131	7, 53	78, 137	-1, 65

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	23	23	11	11	34	34
Mean (SD)	110.6 (15.11)	32.4 (14.99)	113.1 (13.21)	35.5 (14.31)	111.4 (14.37)	33.4 (14.63)
Median	109.0	30.0	116.0	35.0	110.5	33.5
Min, Max	83, 145	4, 73	94, 131	11, 55	83, 145	4, 73
Week 48						
n	18	18	12	12	30	30
Mean (SD)	106.9 (15.65)	30.6 (18.10)	111.9 (12.49)	34.5 (10.32)	108.9 (14.45)	32.2 (15.37)
Median	104.0	31.0	112.5	34.0	108.5	33.0
Min, Max	82, 140	3, 68	85, 129	12, 50	82, 140	3, 68
Week 52						
n	15	15	10	10	25	25
Mean (SD)	109.7 (14.74)	33.5 (14.63)	111.9 (17.42)	35.7 (9.67)	110.6 (15.55)	34.4 (12.69)
Median	109.0	38.0	113.0	33.5	113.0	36.0
Min, Max	76, 130	-3, 56	81, 142	24, 53	76, 142	-3, 56

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB
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Table GERMANY.2.6.2.5

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by the Randomization Stratification Factors of Transfusion History
(> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	22	22	10	10	32	32
Mean (SD)	106.7 (18.86)	29.3 (17.76)	107.9 (15.62)	33.4 (11.77)	107.1 (17.67)	30.6 (16.05)
Median	111.0	32.0	109.0	31.0	110.0	32.0
Min, Max	74, 133	2, 56	81, 128	17, 54	74, 133	2, 56
Week 60						
n	14	14	9	9	23	23
Mean (SD)	108.8 (17.23)	31.9 (17.34)	107.6 (20.74)	30.4 (14.18)	108.3 (18.22)	31.3 (15.85)
Median	111.5	32.5	107.0	30.0	109.0	32.0
Min, Max	77, 137	-2, 65	75, 141	12, 52	75, 141	-2, 65
Week 64						
n	12	12	8	8	20	20
Mean (SD)	107.0 (13.76)	31.2 (13.24)	106.5 (19.54)	32.1 (14.08)	106.8 (15.82)	31.6 (13.22)
Median	106.5	28.0	112.0	33.0	107.5	30.0
Min, Max	79, 131	15, 56	72, 131	9, 57	72, 131	9, 57

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.5

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	10	10	7	7	17	17
Mean (SD)	107.1 (14.38)	30.8 (11.34)	103.6 (20.35)	29.1 (16.17)	105.6 (16.58)	30.1 (13.08)
Median	111.0	33.0	107.0	29.0	108.0	32.0
Min, Max	74, 124	12, 43	66, 132	3, 56	66, 132	3, 56
Week 72						
n	9	9	5	5	14	14
Mean (SD)	109.9 (10.53)	33.1 (9.31)	102.2 (17.28)	24.0 (14.40)	107.1 (13.22)	29.9 (11.73)
Median	110.0	30.0	109.0	24.0	109.0	28.5
Min, Max	91, 123	19, 46	84, 124	2, 40	84, 124	2, 46
Week 80						
n	9	9	4	4	13	13
Mean (SD)	105.7 (17.34)	29.0 (17.44)	103.3 (25.33)	26.8 (15.71)	104.9 (19.03)	28.3 (16.30)
Median	104.0	26.0	111.5	32.0	105.0	29.0
Min, Max	87, 132	8, 59	67, 123	4, 39	67, 132	4, 59

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.5

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	4	4	4	4	8	8
Mean (SD)	113.8 (17.21)	35.5 (14.82)	97.0 (14.83)	22.0 (8.83)	105.4 (17.36)	28.8 (13.40)
Median	119.0	34.0	101.0	20.5	107.0	27.5
Min, Max	89, 128	19, 55	76, 110	13, 34	76, 128	13, 55
Week 96						
n	2	2	2	2	4	4
Mean (SD)	126.5 (2.12)	49.5 (3.54)	93.5 (19.09)	24.0 (9.90)	110.0 (22.05)	36.8 (15.92)
Median	126.5	49.5	93.5	24.0	116.0	39.0
Min, Max	125, 128	47, 52	80, 107	17, 31	80, 128	17, 52
Week 104						
n	0	0	1	1	1	1
Mean (SD)			104.0 (NA)	19.0 (NA)	104.0 (NA)	19.0 (NA)
Median			104.0	19.0	104.0	19.0
Min, Max			104, 104	19, 19	104, 104	19, 19

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		15		50	
Mean (SD)	72.0 (7.67)		72.3 (8.45)		72.1 (7.82)	
Median	73.0		76.0		74.5	
Min, Max	55, 84		54, 83		54, 84	
Week 1						
n	30	30	14	14		
Mean (SD)	95.6 (12.10)	23.2 (10.44)	80.0 (12.89)	7.9 (10.06)		
Median	92.5	21.0	79.0	6.5		
Min, Max	70, 122	2, 45	62, 114	-5, 36		
Week 2						
n	33	33	14	14		
Mean (SD)	102.9 (13.23)	31.1 (12.11)	80.3 (10.26)	8.5 (10.79)		
Median	103.0	27.0	81.0	7.5		
Min, Max	80, 133	4, 54	64, 105	-9, 32		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	26	26	14	14		
Mean (SD)	101.0 (12.42)	30.0 (13.18)	80.7 (12.03)	8.9 (9.60)		
Median	100.5	28.0	83.5	7.5		
Min, Max	82, 126	1, 58	58, 98	-3, 34		
Week 4						
n	33	33	14	14		
Mean (SD)	103.5 (15.60)	31.4 (13.91)	80.7 (13.79)	8.9 (10.72)		
Median	103.0	32.0	83.5	7.5		
Min, Max	74, 139	5, 58	58, 108	-7, 30		
Week 6						
n	30	30	14	14		
Mean (SD)	101.5 (15.49)	30.2 (14.58)	81.3 (9.63)	9.5 (6.57)		
Median	103.5	30.0	82.5	8.0		
Min, Max	73, 133	2, 59	62, 97	0, 19		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	30	30	14	14		
Mean (SD)	99.0 (15.80)	27.5 (17.33)	80.0 (8.87)	7.9 (7.81)		
Median	100.5	28.5	81.0	7.5		
Min, Max	64, 130	-14, 68	66, 96	-9, 21		
Week 10						
n	21	21	12	12		
Mean (SD)	101.9 (14.97)	31.1 (13.87)	78.2 (13.76)	5.1 (10.33)		
Median	98.0	29.0	82.5	5.5		
Min, Max	80, 130	9, 54	51, 96	-20, 20		
Week 12						
n	30	30	14	14		
Mean (SD)	101.4 (16.12)	30.0 (16.10)	80.5 (9.62)	7.5 (10.07)		
Median	99.0	28.0	84.0	8.0		
Min, Max	68, 131	-8, 60	54, 89	-7, 31		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	27	27	13	13		
Mean (SD)	103.0 (17.26)	30.4 (15.15)	93.0 (10.53)	19.4 (8.38)		
Median	99.0	28.0	93.0	18.0		
Min, Max	70, 135	-1, 59	75, 110	4, 32		
Week 14						
n	29	29	12	12		
Mean (SD)	103.1 (19.45)	30.6 (17.21)	103.1 (19.41)	29.7 (16.26)		
Median	103.0	32.0	97.0	26.5		
Min, Max	70, 146	-5, 65	81, 152	10, 72		
Week 15						
n	24	24	12	12		
Mean (SD)	103.0 (17.53)	31.4 (16.89)	102.8 (12.63)	28.3 (9.20)		
Median	99.0	29.0	102.5	27.5		
Min, Max	75, 145	2, 64	85, 126	14, 50		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

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Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	28	28	14	14		
Mean (SD)	102.3 (19.11)	29.9 (17.44)	99.3 (13.42)	27.1 (14.55)		
Median	103.5	28.5	99.0	28.0		
Min, Max	67, 147	-4, 66	74, 120	3, 60		
Week 18						
n	27	27	12	12		
Mean (SD)	97.7 (19.36)	25.6 (20.19)	96.1 (15.26)	21.6 (13.08)		
Median	92.0	24.0	96.5	23.0		
Min, Max	69, 143	-4, 63	73, 118	0, 42		
Week 20						
n	24	24	10	10		
Mean (SD)	102.2 (21.18)	31.0 (18.81)	97.8 (17.80)	23.6 (16.85)		
Median	99.5	32.0	104.0	24.5		
Min, Max	67, 145	-4, 64	66, 123	-5, 49		

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Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	28	28	12	12		
Mean (SD)	105.3 (21.31)	33.0 (20.88)	101.8 (15.07)	29.2 (16.34)		
Median	105.5	33.0	106.5	30.0		
Min, Max	65, 146	-6, 67	70, 121	-1, 53		
Week 24						
n	29	29	11	11		
Mean (SD)	104.6 (19.78)	32.5 (19.13)	99.9 (15.43)	27.1 (14.23)		
Median	104.0	31.0	105.0	29.0		
Min, Max	70, 152	-6, 71	76, 125	3, 51		
Week 28						
n	27	27	10	10	37	37
Mean (SD)	102.4 (21.68)	30.7 (20.55)	100.1 (15.88)	27.8 (14.54)	101.8 (20.09)	29.9 (18.96)
Median	98.0	30.0	98.5	23.5	98.0	28.0
Min, Max	60, 148	-16, 67	64, 124	2, 50	60, 148	-16, 67

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	26	26	10	10	36	36
Mean (SD)	101.7 (19.55)	29.7 (19.34)	98.1 (16.27)	26.0 (14.05)	100.7 (18.54)	28.7 (17.91)
Median	101.0	30.0	97.5	25.5	100.0	28.5
Min, Max	66, 132	-9, 64	66, 122	8, 48	66, 132	-9, 64
Week 36						
n	23	23	11	11	34	34
Mean (SD)	104.9 (18.37)	33.7 (17.16)	104.5 (14.58)	33.2 (19.01)	104.8 (17.02)	33.5 (17.49)
Median	104.0	32.0	104.0	32.0	104.0	32.0
Min, Max	73, 143	-3, 62	86, 124	8, 61	73, 143	-3, 62
Week 40						
n	25	25	10	10	35	35
Mean (SD)	105.6 (20.18)	33.4 (19.59)	98.9 (18.54)	27.0 (17.43)	103.7 (19.69)	31.6 (18.97)
Median	104.0	33.0	99.5	25.5	101.0	33.0
Min, Max	66, 155	-1, 74	57, 122	-5, 53	57, 155	-5, 74

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	23	23	10	10	33	33
Mean (SD)	103.9 (23.26)	31.6 (20.79)	105.1 (12.55)	34.3 (14.82)	104.3 (20.41)	32.4 (18.99)
Median	100.0	34.0	100.5	36.5	100.0	36.0
Min, Max	58, 146	-17, 73	89, 129	13, 55	58, 146	-17, 73
Week 48						
n	18	18	11	11	29	29
Mean (SD)	105.8 (22.53)	35.1 (19.48)	102.5 (14.17)	31.3 (13.24)	104.6 (19.56)	33.7 (17.22)
Median	106.5	34.0	108.0	33.0	108.0	34.0
Min, Max	68, 153	6, 72	82, 123	8, 50	68, 153	6, 72
Week 52						
n	17	17	9	9	26	26
Mean (SD)	104.1 (19.51)	32.4 (15.62)	98.4 (20.09)	28.4 (16.49)	102.1 (19.50)	31.0 (15.72)
Median	107.0	36.0	100.0	29.0	103.5	31.5
Min, Max	62, 143	5, 62	64, 127	2, 53	62, 143	2, 62

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	21	21	10	10	31	31
Mean (SD)	105.1 (22.85)	33.2 (17.72)	100.6 (17.91)	30.0 (16.57)	103.7 (21.19)	32.2 (17.14)
Median	107.0	33.0	98.5	29.5	105.0	32.0
Min, Max	52, 148	-5, 67	78, 128	-3, 54	52, 148	-5, 67
Week 60						
n	15	15	9	9	24	24
Mean (SD)	106.1 (22.31)	34.8 (18.32)	96.0 (15.52)	24.3 (14.03)	102.3 (20.29)	30.9 (17.30)
Median	102.0	33.0	91.0	23.0	97.5	31.0
Min, Max	60, 151	3, 70	75, 126	4, 52	60, 151	3, 70
Week 64						
n	16	16	9	9	25	25
Mean (SD)	103.6 (21.23)	31.0 (16.20)	99.6 (19.63)	27.9 (17.87)	102.2 (20.35)	29.9 (16.52)
Median	103.0	30.0	100.0	33.0	103.0	32.0
Min, Max	47, 137	-10, 56	72, 131	-2, 57	47, 137	-10, 57

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	11	11	7	7	18	18
Mean (SD)	96.6 (21.86)	25.2 (16.33)	97.1 (22.86)	26.4 (20.40)	96.8 (21.58)	25.7 (17.44)
Median	95.0	24.0	92.0	32.0	94.5	27.0
Min, Max	50, 129	-7, 48	66, 132	-3, 56	50, 132	-7, 56
Week 72						
n	10	10	5	5	15	15
Mean (SD)	98.8 (16.29)	26.9 (12.53)	95.2 (20.54)	22.2 (19.68)	97.6 (17.15)	25.3 (14.72)
Median	96.5	27.0	91.0	29.0	96.0	27.0
Min, Max	68, 119	8, 46	70, 122	-11, 39	68, 122	-11, 46
Week 80						
n	7	7	5	5	12	12
Mean (SD)	99.3 (16.94)	26.3 (16.99)	98.4 (19.22)	25.4 (12.10)	98.9 (17.06)	25.9 (14.52)
Median	92.0	19.0	105.0	29.0	95.0	27.5
Min, Max	84, 132	8, 59	67, 114	4, 33	67, 132	4, 59

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	4	4	5	5	9	9
Mean (SD)	98.8 (20.11)	25.8 (19.96)	97.4 (14.10)	21.6 (9.34)	98.0 (15.86)	23.4 (14.06)
Median	92.0	19.0	98.0	22.0	95.0	19.0
Min, Max	83, 128	10, 55	76, 110	12, 34	76, 128	10, 55
Week 96						
n	3	3	4	4	7	7
Mean (SD)	111.0 (17.78)	38.0 (17.78)	105.5 (18.08)	29.8 (9.84)	107.9 (16.66)	33.3 (13.16)
Median	117.0	44.0	110.0	30.5	113.0	31.0
Min, Max	91, 125	18, 52	80, 122	17, 41	80, 125	17, 52
Week 104						
n	0	0	1	1	1	1
Mean (SD)			115.0 (NA)	32.0 (NA)	115.0 (NA)	32.0 (NA)
Median			115.0	32.0	115.0	32.0
Min, Max			115, 115	32, 32	115, 115	32, 32

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		11		33	
Mean (SD)	84.2 (6.96)		88.0 (3.95)		85.5 (6.32)	
Median	85.0		89.0		86.0	
Min, Max	62, 94		83, 93		62, 94	
Week 1						
n	20	20	9	9		
Mean (SD)	103.2 (9.77)	18.7 (9.68)	100.7 (10.07)	12.7 (10.25)		
Median	104.0	19.5	100.0	11.0		
Min, Max	83, 120	-2, 35	84, 121	-5, 35		
Week 2						
n	22	22	9	9		
Mean (SD)	107.2 (9.38)	23.0 (11.24)	99.3 (8.53)	11.3 (6.48)		
Median	111.0	22.5	99.0	12.0		
Min, Max	84, 123	3, 55	86, 111	1, 20		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	20	20	8	8		
Mean (SD)	111.5 (11.89)	27.6 (13.76)	100.3 (12.58)	12.8 (10.90)		
Median	115.0	28.5	100.5	10.5		
Min, Max	80, 125	-1, 59	81, 117	-3, 31		
Week 4						
n	19	19	10	10		
Mean (SD)	115.1 (14.01)	29.2 (13.21)	95.1 (9.96)	7.6 (8.54)		
Median	121.0	31.0	92.5	7.0		
Min, Max	74, 131	-7, 47	79, 114	-5, 21		
Week 6						
n	20	20	10	10		
Mean (SD)	114.8 (9.46)	29.7 (10.85)	93.9 (8.02)	6.4 (7.56)		
Median	117.5	28.5	94.5	7.5		
Min, Max	99, 127	9, 50	81, 107	-5, 16		

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	16	16	10	10		
Mean (SD)	112.4 (12.19)	26.6 (13.74)	97.1 (9.88)	9.6 (9.40)		
Median	114.0	25.5	97.5	11.5		
Min, Max	91, 129	5, 48	76, 112	-7, 26		
Week 10						
n	17	17	10	10		
Mean (SD)	112.9 (10.46)	27.3 (12.88)	92.8 (10.42)	5.3 (8.59)		
Median	113.0	28.0	91.0	4.5		
Min, Max	97, 128	9, 51	78, 106	-7, 19		
Week 12						
n	17	17	10	10		
Mean (SD)	113.9 (8.69)	30.1 (13.06)	94.1 (7.39)	6.6 (6.55)		
Median	114.0	31.0	95.0	7.0		
Min, Max	93, 125	3, 58	80, 106	-3, 21		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	15	15	8	8		
Mean (SD)	112.5 (10.72)	28.5 (14.62)	102.5 (7.65)	16.3 (7.23)		
Median	118.0	28.0	103.5	16.5		
Min, Max	95, 128	4, 58	87, 112	1, 25		
Week 14						
n	16	16	10	10		
Mean (SD)	112.9 (13.07)	29.1 (17.11)	111.5 (11.83)	24.0 (10.69)		
Median	117.5	32.5	112.0	25.5		
Min, Max	90, 131	3, 69	90, 137	4, 44		
Week 15						
n	16	16	7	7		
Mean (SD)	109.3 (12.98)	24.6 (14.13)	114.3 (14.96)	25.7 (13.56)		
Median	113.0	27.0	114.0	29.0		
Min, Max	80, 125	-6, 44	93, 141	7, 48		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	18	18	10	10		
Mean (SD)	112.0 (12.29)	26.5 (14.17)	113.5 (17.29)	26.0 (15.43)		
Median	114.0	26.5	111.0	27.0		
Min, Max	94, 134	2, 53	91, 153	5, 60		
Week 18						
n	13	13	8	8		
Mean (SD)	108.5 (13.68)	23.7 (15.25)	113.5 (13.84)	26.0 (11.87)		
Median	111.0	28.0	112.0	25.0		
Min, Max	83, 127	2, 47	98, 139	11, 46		
Week 20						
n	17	17	9	9		
Mean (SD)	109.9 (12.31)	24.4 (14.05)	106.3 (15.60)	19.0 (14.48)		
Median	110.0	26.0	99.0	14.0		
Min, Max	84, 133	3, 52	94, 136	2, 43		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	15	15	7	7		
Mean (SD)	110.7 (15.20)	25.8 (16.18)	112.7 (18.28)	25.4 (16.44)		
Median	110.0	24.0	114.0	29.0		
Min, Max	84, 132	4, 50	93, 143	8, 50		
Week 24						
n	18	18	7	7		
Mean (SD)	108.1 (15.57)	23.2 (16.68)	111.4 (19.13)	24.6 (16.77)		
Median	104.0	17.5	111.0	26.0		
Min, Max	77, 142	-4, 61	83, 136	0, 45		
Week 28						
n	15	15	8	8	23	23
Mean (SD)	109.5 (13.72)	25.3 (14.77)	106.3 (19.98)	19.5 (18.04)	108.3 (15.79)	23.3 (15.82)
Median	107.0	18.0	102.5	18.0	103.0	18.0
Min, Max	89, 138	3, 57	71, 131	-12, 45	71, 138	-12, 57

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	18	18	8	8	26	26
Mean (SD)	109.0 (14.15)	25.6 (18.18)	105.3 (15.04)	18.6 (14.69)	107.8 (14.23)	23.5 (17.21)
Median	109.0	25.0	103.0	15.0	106.5	21.5
Min, Max	82, 133	-8, 71	81, 132	-2, 48	81, 133	-8, 71
Week 36						
n	12	12	9	9	21	21
Mean (SD)	110.0 (15.99)	26.3 (16.29)	111.8 (10.81)	24.8 (9.24)	110.8 (13.72)	25.7 (13.44)
Median	112.5	30.0	115.0	27.0	114.0	28.0
Min, Max	78, 129	-1, 48	93, 123	10, 39	78, 129	-1, 48
Week 40						
n	12	12	8	8	20	20
Mean (SD)	107.9 (15.81)	25.8 (19.55)	109.6 (15.14)	22.9 (13.82)	108.6 (15.16)	24.7 (17.14)
Median	106.5	24.0	103.5	19.0	106.5	22.5
Min, Max	78, 136	-1, 65	96, 131	7, 47	78, 136	-1, 65

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	14	14	6	6	20	20
Mean (SD)	110.9 (11.97)	28.1 (13.58)	116.0 (13.80)	28.5 (13.13)	112.4 (12.41)	28.2 (13.10)
Median	110.5	28.0	120.5	31.0	112.0	29.0
Min, Max	83, 130	4, 55	94, 131	11, 47	83, 131	4, 55
Week 48						
n	13	13	7	7	20	20
Mean (SD)	105.4 (11.69)	21.3 (13.46)	113.1 (13.99)	25.9 (13.48)	108.1 (12.75)	22.9 (13.30)
Median	104.0	19.0	113.0	29.0	106.5	24.0
Min, Max	82, 126	3, 45	90, 129	4, 43	82, 129	3, 45
Week 52						
n	10	10	7	7	17	17
Mean (SD)	113.2 (17.62)	29.8 (16.63)	115.4 (15.54)	28.0 (14.96)	114.1 (16.33)	29.1 (15.50)
Median	111.5	34.0	113.0	28.0	113.0	32.0
Min, Max	76, 136	-3, 48	92, 142	6, 49	76, 142	-3, 49

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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ALXN2040-PNH-301

Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	11	11	6	6	17	17
Mean (SD)	105.9 (16.94)	21.3 (15.98)	112.0 (8.79)	25.5 (11.91)	108.1 (14.58)	22.8 (14.43)
Median	108.0	18.0	111.5	26.0	109.0	26.0
Min, Max	81, 128	2, 45	100, 125	8, 41	81, 128	2, 45
Week 60						
n	9	9	6	6	15	15
Mean (SD)	106.7 (17.11)	22.4 (15.12)	110.3 (18.89)	22.2 (18.31)	108.1 (17.27)	22.3 (15.82)
Median	112.0	31.0	105.0	18.0	107.0	22.0
Min, Max	77, 125	-2, 37	90, 141	4, 48	77, 141	-2, 48
Week 64						
n	5	5	5	5	10	10
Mean (SD)	107.4 (10.01)	25.2 (11.34)	107.2 (10.96)	21.2 (13.44)	107.3 (9.90)	23.2 (11.91)
Median	107.0	24.0	105.0	20.0	106.5	22.0
Min, Max	93, 121	15, 43	96, 123	7, 39	93, 123	7, 43

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	8	8	5	5	13	13
Mean (SD)	115.5 (7.15)	35.4 (13.22)	104.6 (7.77)	18.6 (9.37)	111.3 (8.97)	28.9 (14.26)
Median	116.0	35.0	107.0	22.0	113.0	29.0
Min, Max	103, 124	12, 59	92, 113	6, 29	92, 124	6, 59
Week 72						
n	6	6	4	4	10	10
Mean (SD)	114.7 (11.88)	34.8 (10.83)	102.3 (17.73)	17.8 (17.75)	109.7 (14.97)	28.0 (15.75)
Median	109.5	36.0	100.0	14.5	109.0	29.0
Min, Max	105, 135	19, 47	85, 124	2, 40	85, 135	2, 47
Week 80						
n	4	4	2	2	6	6
Mean (SD)	108.0 (18.57)	26.0 (19.61)	120.5 (3.54)	37.0 (2.83)	112.2 (15.84)	29.7 (16.27)
Median	106.5	22.5	120.5	37.0	113.5	33.5
Min, Max	87, 132	8, 51	118, 123	35, 39	87, 132	8, 51

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.6

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	5	5	1	1	6	6
Mean (SD)	107.6 (20.18)	28.0 (10.58)	104.0 (NA)	19.0 (NA)	107.0 (18.11)	26.5 (10.15)
Median	115.0	33.0	104.0	19.0	114.0	30.0
Min, Max	72, 122	10, 35	104, 104	19, 19	72, 122	10, 35
Week 96						
n	2	2	0	0	2	2
Mean (SD)	114.5 (19.09)	35.0 (16.97)			114.5 (19.09)	35.0 (16.97)
Median	114.5	35.0			114.5	35.0
Min, Max	101, 128	23, 47			101, 128	23, 47
Week 104						
n	1	1	1	1	2	2
Mean (SD)	109.0 (NA)	31.0 (NA)	104.0 (NA)	19.0 (NA)	106.5 (3.54)	25.0 (8.49)
Median	109.0	31.0	104.0	19.0	106.5	25.0
Min, Max	109, 109	31, 31	104, 104	19, 19	104, 109	19, 31

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.7

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		12		33	
Mean (SD)	79.7 (7.93)		79.5 (10.26)		79.6 (8.69)	
Median	81.0		79.5		81.0	
Min, Max	61, 91		61, 93		61, 93	
Week 1						
n	18	18	9	9		
Mean (SD)	100.7 (12.15)	19.2 (11.42)	87.1 (18.60)	9.1 (11.19)		
Median	104.0	19.0	81.0	10.0		
Min, Max	75, 120	-2, 43	62, 121	-5, 35		
Week 2						
n	21	21	10	10		
Mean (SD)	105.6 (12.35)	26.0 (12.96)	84.9 (13.95)	7.1 (6.24)		
Median	106.0	26.0	83.5	7.5		
Min, Max	84, 126	3, 54	64, 106	-6, 17		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.7

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	15	15	9	9		
Mean (SD)	104.8 (14.06)	23.6 (13.27)	83.8 (13.26)	7.6 (4.93)		
Median	105.0	25.0	84.0	7.0		
Min, Max	80, 125	-1, 41	58, 103	-3, 14		
Week 4						
n	21	21	11	11		
Mean (SD)	107.3 (16.32)	27.6 (14.51)	83.2 (13.27)	4.9 (5.79)		
Median	106.0	30.0	85.0	4.0		
Min, Max	74, 139	-7, 58	58, 100	-5, 15		
Week 6						
n	17	17	11	11		
Mean (SD)	108.1 (12.03)	28.5 (10.31)	85.4 (10.07)	7.1 (7.09)		
Median	108.0	26.0	87.0	8.0		
Min, Max	85, 127	8, 47	62, 101	-5, 18		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.7

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	17	17	10	10		
Mean (SD)	103.1 (13.15)	24.5 (11.48)	85.8 (14.07)	7.3 (9.04)		
Median	101.0	20.0	85.0	6.0		
Min, Max	81, 125	6, 42	67, 112	-7, 26		
Week 10						
n	12	12	10	10		
Mean (SD)	104.6 (10.24)	23.1 (8.03)	85.7 (11.86)	7.2 (8.90)		
Median	104.0	23.0	87.0	7.5		
Min, Max	90, 124	9, 37	62, 105	-4, 20		
Week 12						
n	14	14	10	10		
Mean (SD)	107.0 (11.99)	28.0 (9.17)	82.5 (11.73)	2.7 (5.68)		
Median	109.0	28.0	86.5	2.5		
Min, Max	87, 123	15, 42	54, 97	-7, 11		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.7

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	13	13	10	10		
Mean (SD)	107.5 (11.92)	27.1 (10.96)	94.0 (9.27)	17.1 (7.92)		
Median	104.0	24.0	94.5	15.5		
Min, Max	88, 129	13, 48	76, 105	1, 28		
Week 14						
n	16	16	10	10		
Mean (SD)	108.1 (11.01)	28.7 (10.46)	101.2 (9.94)	22.7 (9.82)		
Median	110.0	32.0	101.5	25.0		
Min, Max	88, 125	8, 42	89, 114	4, 38		
Week 15						
n	14	14	9	9		
Mean (SD)	101.9 (12.17)	22.4 (13.57)	104.8 (12.61)	25.3 (12.85)		
Median	104.0	23.0	104.0	24.0		
Min, Max	80, 120	-6, 42	87, 126	7, 50		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.7

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	18	18	11	11		
Mean (SD)	107.1 (11.13)	27.2 (12.39)	100.6 (11.62)	22.4 (12.50)		
Median	106.0	29.0	95.0	18.0		
Min, Max	89, 130	9, 50	87, 120	5, 44		
Week 18						
n	14	14	9	9		
Mean (SD)	102.1 (15.61)	24.4 (17.02)	100.8 (12.66)	21.7 (12.45)		
Median	103.0	26.5	103.0	23.0		
Min, Max	79, 122	-4, 50	76, 118	0, 42		
Week 20						
n	14	14	8	8		
Mean (SD)	105.9 (15.44)	25.7 (14.69)	98.0 (7.78)	17.3 (14.84)		
Median	107.5	25.5	97.0	11.0		
Min, Max	79, 129	2, 50	84, 109	2, 48		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	16	16	7	7		
Mean (SD)	107.8 (16.61)	28.9 (18.39)	99.7 (12.27)	21.6 (15.76)		
Median	109.5	21.5	97.0	20.0		
Min, Max	81, 131	5, 67	81, 121	5, 47		
Week 24						
n	18	18	7	7		
Mean (SD)	103.3 (15.59)	24.2 (17.32)	99.3 (11.84)	21.6 (14.22)		
Median	100.0	22.0	103.0	16.0		
Min, Max	77, 130	-4, 58	79, 116	3, 44		
Week 28						
n	15	15	8	8	23	23
Mean (SD)	103.6 (11.70)	25.1 (14.21)	103.0 (10.00)	24.3 (14.30)	103.4 (10.91)	24.8 (13.92)
Median	103.0	18.0	100.0	21.0	101.0	20.0
Min, Max	91, 129	11, 56	93, 124	7, 50	91, 129	7, 56

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	15	15	9	9	24	24
Mean (SD)	106.7 (17.33)	25.6 (18.45)	100.9 (8.64)	20.7 (12.01)	104.5 (14.74)	23.8 (16.22)
Median	106.0	23.0	99.0	15.0	103.5	21.5
Min, Max	66, 130	-9, 58	86, 118	10, 45	66, 130	-9, 58
Week 36						
n	14	14	9	9	23	23
Mean (SD)	108.1 (15.28)	29.6 (17.12)	106.9 (12.32)	29.9 (18.11)	107.7 (13.91)	29.7 (17.10)
Median	110.0	29.0	106.0	19.0	107.0	28.0
Min, Max	82, 130	-1, 58	86, 124	10, 61	82, 130	-1, 61
Week 40						
n	15	15	9	9	24	24
Mean (SD)	104.5 (17.02)	26.3 (18.87)	101.9 (10.04)	23.4 (14.69)	103.5 (14.60)	25.3 (17.14)
Median	105.0	24.0	98.0	22.0	100.0	23.0
Min, Max	76, 137	-1, 65	89, 122	7, 53	76, 137	-1, 65

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	16	16	7	7	23	23
Mean (SD)	108.8 (20.63)	29.9 (20.31)	104.4 (11.03)	28.6 (16.44)	107.5 (18.10)	29.5 (18.85)
Median	109.5	29.0	105.0	26.0	108.0	28.0
Min, Max	58, 145	-17, 73	89, 120	11, 53	58, 145	-17, 73
Week 48						
n	12	12	9	9	21	21
Mean (SD)	103.3 (17.06)	24.9 (20.18)	104.6 (10.10)	27.6 (14.84)	103.8 (14.19)	26.0 (17.72)
Median	100.5	15.0	108.0	32.0	102.0	29.0
Min, Max	79, 140	6, 68	89, 116	4, 50	79, 140	4, 68
Week 52						
n	11	11	9	9	20	20
Mean (SD)	106.4 (14.07)	28.0 (13.08)	106.2 (10.49)	27.4 (11.60)	106.3 (12.27)	27.8 (12.11)
Median	107.0	27.0	105.0	29.0	105.5	28.0
Min, Max	88, 130	11, 56	92, 125	6, 42	88, 130	6, 56

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	12	12	10	10	22	22
Mean (SD)	106.3 (13.63)	28.3 (15.07)	106.1 (12.29)	27.6 (12.60)	106.2 (12.73)	28.0 (13.68)
Median	105.0	28.5	106.5	26.5	106.5	27.0
Min, Max	88, 126	10, 53	84, 124	8, 46	84, 126	8, 53
Week 60						
n	10	10	8	8	18	18
Mean (SD)	107.2 (16.61)	29.9 (18.31)	96.0 (10.84)	15.9 (9.73)	102.2 (15.07)	23.7 (16.37)
Median	102.0	27.0	98.0	14.0	100.5	22.0
Min, Max	89, 137	7, 65	75, 109	4, 33	75, 137	4, 65
Week 64						
n	7	7	8	8	15	15
Mean (SD)	105.1 (13.97)	27.7 (11.44)	101.3 (15.13)	21.9 (12.98)	103.1 (14.22)	24.6 (12.22)
Median	110.0	24.0	105.5	22.0	110.0	24.0
Min, Max	79, 121	15, 48	72, 116	7, 36	72, 121	7, 48

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	8	8	7	7	15	15
Mean (SD)	101.8 (15.64)	24.9 (11.22)	103.6 (20.57)	23.7 (18.61)	102.6 (17.45)	24.3 (14.55)
Median	105.5	26.0	107.0	24.0	107.0	24.0
Min, Max	74, 118	12, 40	66, 132	3, 56	66, 132	3, 56
Week 72						
n	5	5	5	5	10	10
Mean (SD)	107.2 (10.28)	30.2 (10.85)	98.2 (16.66)	20.0 (16.43)	102.7 (13.89)	25.1 (14.18)
Median	110.0	27.0	91.0	21.0	103.0	25.0
Min, Max	96, 119	19, 46	84, 122	2, 39	84, 122	2, 46
Week 80						
n	4	4	4	4	8	8
Mean (SD)	104.3 (19.60)	27.5 (21.19)	100.5 (22.96)	24.3 (13.79)	102.4 (19.86)	25.9 (16.64)
Median	98.0	19.0	108.5	29.0	104.5	24.0
Min, Max	89, 132	13, 59	67, 118	4, 35	67, 132	4, 59

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	4	4	4	4	8	8
Mean (SD)	105.5 (22.78)	29.3 (19.60)	98.5 (16.03)	24.0 (8.83)	102.0 (18.62)	26.6 (14.35)
Median	105.5	26.0	104.0	24.5	104.0	24.5
Min, Max	83, 128	10, 55	76, 110	13, 34	76, 128	10, 55
Week 96						
n	2	2	3	3	5	5
Mean (SD)	108.0 (24.04)	35.0 (24.04)	100.0 (17.58)	26.0 (7.81)	103.2 (17.84)	29.6 (14.12)
Median	108.0	35.0	107.0	30.0	107.0	30.0
Min, Max	91, 125	18, 52	80, 113	17, 31	80, 125	17, 52
Week 104						
n	0	0	1	1	1	1
Mean (SD)			115.0 (NA)	32.0 (NA)	115.0 (NA)	32.0 (NA)
Median			115.0	32.0	115.0	32.0
Min, Max			115, 115	32, 32	115, 115	32, 32

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		14		50	
Mean (SD)	75.0 (9.97)		78.5 (10.91)		76.0 (10.25)	
Median	76.5		80.5		77.5	
Min, Max	55, 94		54, 93		54, 94	
Week 1						
n	32	32	14	14		
Mean (SD)	97.4 (11.52)	22.6 (9.57)	88.7 (13.95)	10.2 (9.89)		
Median	98.0	21.0	85.5	10.0		
Min, Max	70, 122	2, 45	62, 114	-5, 36		
Week 2						
n	34	34	13	13		
Mean (SD)	104.0 (11.82)	29.0 (11.96)	89.9 (13.05)	11.5 (10.92)		
Median	105.0	26.5	86.0	12.0		
Min, Max	80, 133	8, 55	72, 111	-9, 32		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	31	31	13	13		
Mean (SD)	105.9 (12.90)	31.5 (12.80)	90.6 (16.52)	12.2 (12.23)		
Median	105.0	29.0	90.0	10.0		
Min, Max	82, 126	13, 59	64, 117	-3, 34		
Week 4						
n	31	31	13	13		
Mean (SD)	108.0 (15.93)	32.6 (12.73)	89.7 (14.60)	11.3 (11.48)		
Median	111.0	33.0	89.0	14.0		
Min, Max	74, 130	10, 56	67, 114	-7, 30		
Week 6						
n	33	33	13	13		
Mean (SD)	106.1 (16.21)	30.7 (14.42)	87.5 (11.79)	9.2 (7.09)		
Median	107.0	32.0	90.0	8.0		
Min, Max	73, 133	2, 59	70, 107	-1, 19		

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and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	29	29	14	14		
Mean (SD)	104.0 (17.48)	28.7 (18.18)	88.1 (11.75)	9.6 (8.03)		
Median	104.0	32.0	90.5	11.0		
Min, Max	64, 130	-14, 68	66, 106	-9, 21		
Week 10						
n	26	26	12	12		
Mean (SD)	107.8 (15.67)	32.3 (14.47)	84.1 (16.37)	3.5 (9.77)		
Median	113.5	32.5	85.0	4.0		
Min, Max	80, 130	9, 54	51, 106	-20, 15		
Week 12						
n	33	33	14	14		
Mean (SD)	105.5 (16.34)	30.9 (16.83)	88.8 (10.02)	10.3 (9.14)		
Median	112.0	31.0	89.0	9.5		
Min, Max	68, 131	-8, 60	69, 106	-5, 31		

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.2.7

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	29	29	11	11		
Mean (SD)	105.9 (17.42)	30.9 (16.28)	99.0 (11.32)	19.2 (8.18)		
Median	112.0	32.0	100.0	18.0		
Min, Max	70, 135	-1, 59	75, 112	4, 32		
Week 14						
n	29	29	12	12		
Mean (SD)	105.7 (20.94)	30.8 (19.84)	111.7 (19.77)	30.8 (16.21)		
Median	112.0	32.0	111.5	26.0		
Min, Max	70, 146	-5, 69	81, 152	10, 72		
Week 15						
n	26	26	10	10		
Mean (SD)	107.5 (17.61)	32.1 (16.42)	109.1 (16.05)	29.2 (8.65)		
Median	112.0	33.0	110.0	29.5		
Min, Max	75, 145	2, 64	85, 141	14, 48		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.7

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	28	28	13	13		
Mean (SD)	105.4 (20.49)	29.5 (18.34)	109.1 (19.23)	30.2 (15.73)		
Median	106.0	28.5	110.0	27.0		
Min, Max	67, 147	-4, 66	74, 153	3, 60		
Week 18						
n	26	26	11	11		
Mean (SD)	100.8 (19.83)	25.2 (19.65)	104.9 (19.98)	24.7 (12.94)		
Median	98.5	23.5	109.0	27.0		
Min, Max	69, 143	-4, 63	73, 139	2, 46		
Week 20						
n	27	27	11	11		
Mean (SD)	105.1 (19.85)	29.6 (18.38)	104.6 (21.25)	24.5 (15.98)		
Median	109.0	32.0	106.0	26.0		
Min, Max	67, 145	-4, 64	66, 136	-5, 49		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.7

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	27	27	12	12		
Mean (SD)	106.9 (21.15)	31.4 (20.38)	109.4 (18.36)	31.4 (15.68)		
Median	110.0	33.0	110.5	30.5		
Min, Max	65, 146	-6, 66	70, 143	-1, 53		
Week 24						
n	29	29	11	11		
Mean (SD)	107.6 (19.71)	31.9 (19.07)	107.6 (20.01)	29.0 (15.13)		
Median	105.0	31.0	111.0	30.0		
Min, Max	70, 152	-6, 71	76, 136	0, 51		
Week 28						
n	27	27	10	10	37	37
Mean (SD)	105.7 (22.69)	30.8 (20.73)	102.7 (22.44)	24.0 (18.42)	104.9 (22.35)	28.9 (20.11)
Median	107.0	30.0	103.5	26.5	105.0	30.0
Min, Max	60, 148	-16, 67	64, 131	-12, 45	60, 148	-16, 67

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.7

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	29	29	9	9	38	38
Mean (SD)	103.6 (18.15)	29.3 (19.14)	101.7 (21.20)	24.8 (16.95)	103.1 (18.63)	28.2 (18.52)
Median	102.0	29.0	106.0	27.0	104.0	28.5
Min, Max	68, 133	-8, 71	66, 132	-2, 48	66, 133	-8, 71
Week 36						
n	21	21	11	11	32	32
Mean (SD)	105.7 (19.18)	32.2 (17.25)	108.5 (14.49)	29.0 (14.21)	106.6 (17.52)	31.1 (16.11)
Median	109.0	32.0	115.0	31.0	110.0	32.0
Min, Max	73, 143	-3, 62	89, 123	8, 50	73, 143	-3, 62
Week 40						
n	22	22	9	9	31	31
Mean (SD)	107.7 (20.02)	34.1 (19.95)	105.4 (23.30)	26.9 (17.20)	107.0 (20.65)	32.0 (19.20)
Median	107.5	33.0	109.0	32.0	108.0	33.0
Min, Max	66, 155	-1, 74	57, 131	-5, 47	57, 155	-5, 74

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.7

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	21	21	9	9	30	30
Mean (SD)	104.8 (19.56)	30.5 (17.06)	112.9 (15.01)	34.9 (12.17)	107.2 (18.44)	31.8 (15.68)
Median	106.0	34.0	120.0	35.0	106.5	34.0
Min, Max	65, 146	-3, 65	96, 131	16, 55	65, 146	-3, 65
Week 48						
n	19	19	9	9	28	28
Mean (SD)	107.2 (19.66)	32.1 (17.00)	108.8 (18.59)	30.8 (12.04)	107.7 (19.00)	31.7 (15.36)
Median	109.0	34.0	113.0	31.0	111.0	32.0
Min, Max	68, 153	3, 72	82, 129	8, 49	68, 153	3, 72
Week 52						
n	16	16	7	7	23	23
Mean (SD)	108.2 (22.23)	33.8 (17.34)	105.4 (28.68)	29.3 (20.12)	107.3 (23.72)	32.4 (17.89)
Median	113.5	39.5	113.0	28.0	113.0	38.0
Min, Max	62, 143	-3, 62	64, 142	2, 53	62, 143	-3, 62

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.7

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	20	20	6	6	26	26
Mean (SD)	104.9 (24.34)	29.6 (19.67)	102.8 (21.74)	29.5 (19.04)	104.4 (23.35)	29.5 (19.15)
Median	110.5	31.5	102.5	29.5	109.5	31.0
Min, Max	52, 148	-5, 67	78, 128	-3, 54	52, 148	-5, 67
Week 60						
n	14	14	7	7	21	21
Mean (SD)	105.6 (22.90)	30.4 (18.32)	108.3 (22.60)	32.1 (16.42)	106.5 (22.26)	31.0 (17.32)
Median	108.0	33.0	107.0	30.0	107.0	32.0
Min, Max	60, 151	-2, 70	84, 141	4, 52	60, 151	-2, 70
Week 64						
n	14	14	6	6	20	20
Mean (SD)	104.2 (21.56)	30.6 (17.00)	103.7 (20.65)	30.3 (19.99)	104.1 (20.75)	30.5 (17.40)
Median	103.0	28.0	102.5	34.0	103.0	29.0
Min, Max	47, 137	-10, 56	79, 131	-2, 57	47, 137	-10, 57

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.7

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	11	11	5	5	16	16
Mean (SD)	106.6 (22.28)	32.8 (17.89)	95.6 (14.26)	22.4 (15.37)	103.2 (20.32)	29.6 (17.35)
Median	113.0	36.0	92.0	26.0	108.0	33.0
Min, Max	50, 129	-7, 59	78, 113	-3, 38	50, 129	-7, 59
Week 72						
n	11	11	4	4	15	15
Mean (SD)	103.6 (18.90)	29.7 (13.28)	98.5 (23.30)	20.5 (22.04)	102.3 (19.41)	27.3 (15.75)
Median	106.0	28.0	100.0	26.5	106.0	28.0
Min, Max	68, 135	8, 47	70, 124	-11, 40	68, 135	-11, 47
Week 80						
n	7	7	3	3	10	10
Mean (SD)	101.4 (17.20)	25.4 (15.96)	110.3 (14.84)	34.7 (3.79)	104.1 (16.27)	28.2 (13.89)
Median	96.0	26.0	114.0	33.0	102.5	32.0
Min, Max	84, 132	8, 51	94, 123	32, 39	84, 132	8, 51

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.7

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	5	5	2	2	7	7
Mean (SD)	102.2 (18.94)	25.2 (10.78)	98.5 (7.78)	15.5 (4.95)	101.1 (15.89)	22.4 (10.20)
Median	113.0	27.0	98.5	15.5	104.0	19.0
Min, Max	72, 116	10, 35	93, 104	12, 19	72, 116	10, 35
Week 96						
n	3	3	1	1	4	4
Mean (SD)	115.3 (13.58)	38.0 (13.08)	122.0 (NA)	41.0 (NA)	117.0 (11.58)	38.8 (10.78)
Median	117.0	44.0	122.0	41.0	119.5	42.5
Min, Max	101, 128	23, 47	122, 122	41, 41	101, 128	23, 47
Week 104						
n	1	1	1	1	2	2
Mean (SD)	109.0 (NA)	31.0 (NA)	104.0 (NA)	19.0 (NA)	106.5 (3.54)	25.0 (8.49)
Median	109.0	31.0	104.0	19.0	106.5	25.0
Min, Max	109, 109	31, 31	104, 104	19, 19	104, 109	19, 31

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.8

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	68.4 (10.39)		82.0 (7.07)		72.9 (11.29)	
Median	69.5		81.5		75.0	
Min, Max	55, 81		74, 91		55, 91	
Week 1						
n	7	7	4	4		
Mean (SD)	86.9 (10.38)	19.6 (7.07)	88.8 (10.05)	6.8 (3.86)		
Median	88.0	19.0	88.5	6.5		
Min, Max	70, 103	12, 33	78, 100	3, 11		
Week 2						
n	7	7	3	3		
Mean (SD)	94.7 (10.61)	27.4 (8.62)	90.3 (11.72)	7.7 (4.51)		
Median	98.0	26.0	95.0	8.0		
Min, Max	80, 106	19, 46	77, 99	3, 12		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.8

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	7	7	3	3		
Mean (SD)	96.1 (11.74)	28.9 (11.95)	87.7 (11.68)	5.0 (3.46)		
Median	92.0	26.0	90.0	7.0		
Min, Max	83, 117	15, 50	75, 98	1, 7		
Week 4						
n	7	7	3	3		
Mean (SD)	100.0 (13.87)	32.7 (13.85)	81.3 (12.42)	-1.3 (6.03)		
Median	98.0	28.0	88.0	-2.0		
Min, Max	83, 121	12, 56	67, 89	-7, 5		
Week 6						
n	8	8	3	3		
Mean (SD)	95.4 (19.54)	27.0 (19.02)	93.0 (5.20)	10.3 (9.81)		
Median	96.5	23.5	90.0	16.0		
Min, Max	73, 133	2, 52	90, 99	-1, 16		

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB
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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	7	7	4	4		
Mean (SD)	93.7 (9.83)	27.1 (16.13)	86.8 (9.18)	4.8 (5.56)		
Median	100.0	32.0	87.0	2.5		
Min, Max	80, 103	4, 48	77, 96	1, 13		
Week 10						
n	6	6	4	4		
Mean (SD)	95.3 (13.20)	30.2 (14.06)	87.3 (13.82)	5.3 (6.85)		
Median	92.0	29.5	85.0	3.5		
Min, Max	80, 114	9, 53	73, 106	-1, 15		
Week 12						
n	8	8	4	4		
Mean (SD)	94.5 (20.77)	26.1 (22.01)	84.8 (13.38)	2.8 (6.85)		
Median	94.0	24.5	85.5	3.5		
Min, Max	68, 131	-8, 60	69, 99	-5, 9		

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	8	8	4	4		
Mean (SD)	97.6 (20.22)	29.3 (19.81)	98.0 (10.95)	16.0 (3.92)		
Median	93.5	27.5	97.0	15.5		
Min, Max	70, 135	-1, 59	86, 112	12, 21		
Week 14						
n	8	8	4	4		
Mean (SD)	99.3 (23.10)	30.9 (21.60)	118.8 (24.53)	36.8 (23.94)		
Median	90.0	30.0	115.0	28.0		
Min, Max	74, 146	3, 65	93, 152	19, 72		
Week 15						
n	8	8	3	3		
Mean (SD)	99.0 (23.57)	30.6 (22.84)	109.3 (12.01)	27.7 (4.04)		
Median	91.5	28.5	110.0	30.0		
Min, Max	75, 145	2, 64	97, 121	23, 30		

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	8	8	4	4		
Mean (SD)	96.6 (26.18)	28.3 (25.53)	110.5 (8.06)	28.5 (3.70)		
Median	87.0	27.0	108.0	29.0		
Min, Max	67, 147	-4, 66	104, 122	24, 32		
Week 18						
n	8	8	4	4		
Mean (SD)	95.9 (25.01)	27.5 (24.42)	110.8 (9.91)	28.8 (8.34)		
Median	88.0	24.5	109.5	31.0		
Min, Max	69, 143	-2, 63	100, 124	17, 36		
Week 20						
n	8	8	4	4		
Mean (SD)	95.8 (25.58)	27.4 (22.77)	111.5 (11.24)	29.5 (14.53)		
Median	92.5	29.0	113.0	27.5		
Min, Max	67, 145	-4, 64	97, 123	14, 49		

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	8	8	4	4		
Mean (SD)	101.0 (26.99)	32.6 (26.53)	110.3 (12.82)	28.3 (14.71)		
Median	96.5	33.0	114.0	28.5		
Min, Max	65, 146	-6, 66	93, 120	10, 46		
Week 24						
n	7	7	4	4		
Mean (SD)	104.3 (26.62)	36.3 (24.39)	109.3 (19.47)	27.3 (21.06)		
Median	98.0	32.0	114.5	29.0		
Min, Max	70, 152	-6, 71	83, 125	0, 51		
Week 28						
n	6	6	4	4	10	10
Mean (SD)	97.3 (31.95)	31.3 (30.76)	102.3 (21.85)	20.3 (22.51)	99.3 (27.07)	26.9 (26.97)
Median	93.5	30.0	109.0	26.5	102.0	28.5
Min, Max	60, 148	-16, 67	71, 120	-12, 40	60, 148	-16, 67

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	8	8	3	3	11	11
Mean (SD)	98.0 (23.26)	29.6 (23.16)	107.0 (22.61)	24.3 (25.11)	100.5 (22.33)	28.2 (22.53)
Median	95.5	32.0	118.0	27.0	101.0	31.0
Min, Max	68, 132	-3, 64	81, 122	-2, 48	68, 132	-3, 64
Week 36						
n	7	7	3	3	10	10
Mean (SD)	100.6 (23.99)	32.6 (21.77)	112.3 (16.74)	29.7 (19.04)	104.1 (21.87)	31.7 (19.96)
Median	102.0	30.0	122.0	31.0	106.0	30.5
Min, Max	73, 143	-3, 62	93, 122	10, 48	73, 143	-3, 62
Week 40						
n	6	6	3	3	9	9
Mean (SD)	108.0 (29.83)	41.3 (22.83)	112.7 (14.57)	30.0 (16.09)	109.6 (24.79)	37.6 (20.56)
Median	108.0	36.0	119.0	32.0	115.0	33.0
Min, Max	66, 155	9, 74	96, 123	13, 45	66, 155	9, 74

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	5	5	2	2	7	7
Mean (SD)	100.0 (30.36)	31.0 (21.59)	125.0 (5.66)	42.5 (17.68)	107.1 (27.72)	34.3 (19.86)
Median	93.0	30.0	125.0	42.5	110.0	30.0
Min, Max	65, 146	8, 65	121, 129	30, 55	65, 146	8, 65
Week 48						
n	4	4	2	2	6	6
Mean (SD)	107.5 (36.12)	38.3 (25.20)	121.5 (2.12)	39.0 (14.14)	112.2 (28.91)	38.5 (20.52)
Median	104.5	35.0	121.5	39.0	118.0	35.0
Min, Max	68, 153	11, 72	120, 123	29, 49	68, 153	11, 72
Week 52						
n	4	4	1	1	5	5
Mean (SD)	98.0 (35.24)	28.8 (24.03)	127.0 (NA)	53.0 (NA)	103.8 (33.16)	33.6 (23.47)
Median	93.5	24.0	127.0	53.0	107.0	27.0
Min, Max	62, 143	5, 62	127, 127	53, 53	62, 143	5, 62

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Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	4	4	1	1	5	5
Mean (SD)	98.8 (39.98)	29.5 (29.46)	128.0 (NA)	54.0 (NA)	104.6 (37.01)	34.4 (27.76)
Median	97.5	28.0	128.0	54.0	107.0	29.0
Min, Max	52, 148	-5, 67	128, 128	54, 54	52, 148	-5, 67
Week 60						
n	4	4	1	1	5	5
Mean (SD)	102.0 (37.48)	32.8 (28.28)	126.0 (NA)	52.0 (NA)	106.8 (34.19)	36.6 (25.96)
Median	98.5	29.0	126.0	52.0	102.0	36.0
Min, Max	60, 151	3, 70	126, 126	52, 52	60, 151	3, 70
Week 64						
n	4	4	1	1	5	5
Mean (SD)	96.8 (38.13)	27.5 (27.44)	131.0 (NA)	57.0 (NA)	103.6 (36.40)	33.4 (27.18)
Median	101.5	32.0	131.0	57.0	112.0	32.0
Min, Max	47, 137	-10, 56	131, 131	57, 57	47, 137	-10, 57

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Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	3	3	0	0	3	3
Mean (SD)	95.7 (40.92)	23.0 (27.84)			95.7 (40.92)	23.0 (27.84)
Median	108.0	28.0			108.0	28.0
Min, Max	50, 129	-7, 48			50, 129	-7, 48
Week 72						
n	1	1	0	0	1	1
Mean (SD)	68.0 (NA)	11.0 (NA)			68.0 (NA)	11.0 (NA)
Median	68.0	11.0			68.0	11.0
Min, Max	68, 68	11, 11			68, 68	11, 11

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	49		22		71	
Mean (SD)	78.1 (8.68)		78.4 (10.95)		78.2 (9.37)	
Median	78.0		79.5		78.0	
Min, Max	60, 94		54, 93		54, 94	
Week 1						
n	43	43	19	19		
Mean (SD)	100.5 (10.88)	21.7 (10.76)	87.9 (16.68)	10.4 (11.03)		
Median	101.0	20.0	84.0	11.0		
Min, Max	75, 122	-2, 45	62, 121	-5, 36		
Week 2						
n	48	48	20	20		
Mean (SD)	106.1 (11.52)	27.9 (12.84)	87.4 (13.84)	9.9 (9.84)		
Median	106.5	27.0	85.0	8.5		
Min, Max	84, 133	3, 55	64, 111	-9, 32		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	39	39	19	19		
Mean (SD)	107.2 (12.79)	29.0 (13.72)	87.8 (16.09)	11.2 (10.49)		
Median	107.0	28.0	88.0	10.0		
Min, Max	80, 126	-1, 59	58, 117	-3, 34		
Week 4						
n	45	45	21	21		
Mean (SD)	108.9 (16.03)	30.3 (13.65)	87.5 (14.43)	9.8 (9.40)		
Median	111.0	32.0	90.0	9.0		
Min, Max	74, 139	-7, 58	58, 114	-5, 30		
Week 6						
n	42	42	21	21		
Mean (SD)	109.0 (12.92)	30.5 (11.88)	85.6 (11.21)	7.9 (6.80)		
Median	112.0	29.5	87.0	8.0		
Min, Max	80, 127	8, 59	62, 107	-5, 19		

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.6.2.8

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	39	39	20	20		
Mean (SD)	105.4 (16.17)	27.2 (16.21)	87.2 (13.28)	9.4 (8.70)		
Median	106.0	26.0	89.0	10.0		
Min, Max	64, 130	-14, 68	66, 112	-9, 26		
Week 10						
n	32	32	18	18		
Mean (SD)	108.9 (13.41)	29.3 (13.50)	84.3 (14.60)	5.2 (10.00)		
Median	111.5	27.5	87.0	6.5		
Min, Max	81, 130	9, 54	51, 105	-20, 20		
Week 12						
n	39	39	20	20		
Mean (SD)	108.3 (12.73)	30.8 (13.28)	86.5 (10.83)	8.0 (8.82)		
Median	113.0	31.0	87.0	7.0		
Min, Max	77, 125	3, 58	54, 106	-7, 31		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.8

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	34	34	17	17		
Mean (SD)	108.5 (14.15)	29.8 (13.75)	96.3 (10.66)	18.7 (8.62)		
Median	113.5	29.5	99.0	18.0		
Min, Max	80, 130	4, 58	75, 110	1, 32		
Week 14						
n	37	37	18	18		
Mean (SD)	108.1 (16.58)	29.9 (16.19)	104.3 (13.93)	24.9 (10.67)		
Median	110.0	33.0	105.0	25.0		
Min, Max	70, 138	-5, 69	81, 137	4, 45		
Week 15						
n	32	32	16	16		
Mean (SD)	107.2 (13.51)	28.2 (14.26)	106.6 (14.99)	27.3 (11.64)		
Median	109.5	30.0	107.0	27.5		
Min, Max	80, 135	-6, 58	85, 141	7, 50		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.8

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	38	38	20	20		
Mean (SD)	108.1 (14.53)	28.7 (13.94)	104.2 (17.60)	26.3 (15.97)		
Median	107.0	30.0	104.0	27.0		
Min, Max	78, 137	2, 53	74, 153	3, 60		
Week 18						
n	32	32	16	16		
Mean (SD)	102.6 (16.41)	24.3 (17.21)	101.1 (17.82)	22.0 (13.18)		
Median	101.5	24.5	100.5	21.5		
Min, Max	73, 127	-4, 50	73, 139	0, 46		
Week 20						
n	33	33	15	15		
Mean (SD)	107.7 (15.66)	28.5 (15.90)	99.3 (17.51)	19.3 (15.52)		
Median	110.0	28.0	99.0	14.0		
Min, Max	76, 133	2, 54	66, 136	-5, 48		

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.8

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	35	35	15	15		
Mean (SD)	108.6 (17.41)	30.0 (17.96)	104.7 (17.80)	27.7 (16.84)		
Median	110.0	32.0	106.0	29.0		
Min, Max	74, 132	4, 67	70, 143	-1, 53		
Week 24						
n	40	40	14	14		
Mean (SD)	106.2 (16.75)	27.7 (17.48)	103.0 (17.29)	25.8 (13.59)		
Median	104.0	27.0	104.0	27.5		
Min, Max	70, 142	-4, 61	76, 136	3, 45		
Week 28						
n	36	36	14	14	50	50
Mean (SD)	106.2 (16.76)	28.3 (16.50)	103.0 (17.09)	25.2 (14.91)	105.3 (16.74)	27.5 (15.98)
Median	104.0	25.5	100.0	21.0	103.0	22.0
Min, Max	67, 142	0, 58	64, 131	2, 50	64, 142	0, 58

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.2.8

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	36	36	15	15	51	51
Mean (SD)	106.1 (16.31)	27.7 (18.03)	100.1 (14.77)	22.4 (12.70)	104.4 (15.96)	26.1 (16.69)
Median	107.0	26.0	99.0	20.0	105.0	23.0
Min, Max	66, 133	-9, 71	66, 132	8, 48	66, 133	-9, 71
Week 36						
n	28	28	17	17	45	45
Mean (SD)	108.2 (15.71)	30.8 (16.05)	106.9 (12.97)	29.4 (15.66)	107.7 (14.60)	30.2 (15.74)
Median	110.5	32.0	106.0	30.0	109.0	31.0
Min, Max	78, 130	-1, 58	86, 124	8, 61	78, 130	-1, 61
Week 40						
n	31	31	15	15	46	46
Mean (SD)	106.1 (16.43)	29.0 (18.72)	101.9 (17.91)	24.2 (15.91)	104.7 (16.84)	27.4 (17.82)
Median	106.0	28.0	98.0	24.0	104.0	26.5
Min, Max	76, 137	-1, 65	57, 131	-5, 53	57, 137	-5, 65

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.8

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	32	32	14	14	46	46
Mean (SD)	107.6 (18.18)	30.1 (18.09)	106.9 (13.04)	30.6 (13.60)	107.4 (16.64)	30.3 (16.70)
Median	108.5	29.0	103.5	33.5	106.5	31.0
Min, Max	58, 145	-17, 73	89, 131	11, 53	58, 145	-17, 73
Week 48						
n	27	27	16	16	43	43
Mean (SD)	105.4 (15.62)	28.0 (17.31)	104.8 (14.52)	27.9 (13.04)	105.2 (15.05)	28.0 (15.69)
Median	104.0	28.0	109.5	31.5	105.0	29.0
Min, Max	79, 140	3, 68	82, 129	4, 50	79, 140	3, 68
Week 52						
n	23	23	15	15	38	38
Mean (SD)	109.1 (15.47)	31.9 (14.57)	104.5 (19.53)	26.6 (14.31)	107.3 (17.09)	29.8 (14.51)
Median	109.0	36.0	105.0	28.0	108.0	32.5
Min, Max	76, 136	-3, 56	64, 142	2, 49	64, 142	-3, 56

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.8

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	28	28	15	15	43	43
Mean (SD)	106.4 (17.55)	29.0 (16.39)	103.3 (15.10)	26.6 (13.50)	105.3 (16.62)	28.2 (15.33)
Median	108.5	30.5	106.0	27.0	107.0	30.0
Min, Max	74, 133	2, 56	78, 125	-3, 46	74, 133	-3, 56
Week 60						
n	20	20	14	14	34	34
Mean (SD)	107.2 (16.24)	29.7 (16.14)	100.0 (17.14)	21.4 (13.57)	104.2 (16.74)	26.3 (15.48)
Median	108.0	32.0	98.0	22.0	102.5	29.5
Min, Max	77, 137	-2, 65	75, 141	4, 48	75, 141	-2, 65
Week 64						
n	17	17	13	13	30	30
Mean (SD)	106.4 (12.65)	30.1 (11.98)	100.1 (15.45)	23.1 (14.00)	103.6 (14.04)	27.1 (13.15)
Median	106.0	28.0	100.0	30.0	104.0	28.0
Min, Max	79, 131	15, 56	72, 123	-2, 39	72, 131	-2, 56

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.8

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	16	16	12	12	28	28
Mean (SD)	106.3 (14.50)	30.7 (13.35)	100.3 (17.93)	23.2 (16.59)	103.7 (16.03)	27.5 (15.02)
Median	111.0	33.0	105.5	25.0	107.0	29.0
Min, Max	74, 124	12, 59	66, 132	-3, 56	66, 132	-3, 59
Week 72						
n	15	15	9	9	24	24
Mean (SD)	107.2 (13.62)	31.1 (11.51)	98.3 (18.51)	20.2 (17.81)	103.9 (15.85)	27.0 (14.84)
Median	107.0	28.0	91.0	24.0	106.5	27.5
Min, Max	84, 135	8, 47	70, 124	-11, 40	70, 135	-11, 47
Week 80						
n	11	11	7	7	18	18
Mean (SD)	102.5 (17.17)	26.2 (16.99)	104.7 (19.09)	28.7 (11.44)	103.3 (17.42)	27.2 (14.75)
Median	96.0	19.0	112.0	32.0	104.5	29.0
Min, Max	84, 132	8, 59	67, 123	4, 39	67, 132	4, 59

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.8

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	9	9	6	6	15	15
Mean (SD)	103.7 (19.42)	27.0 (14.38)	98.5 (12.90)	21.2 (8.42)	101.6 (16.78)	24.7 (12.34)
Median	113.0	27.0	101.0	20.5	104.0	22.0
Min, Max	72, 128	10, 55	76, 110	12, 34	72, 128	10, 55
Week 96						
n	5	5	4	4	9	9
Mean (SD)	112.4 (15.90)	36.8 (15.25)	105.5 (18.08)	29.8 (9.84)	109.3 (16.19)	33.7 (12.90)
Median	117.0	44.0	110.0	30.5	113.0	31.0
Min, Max	91, 128	18, 52	80, 122	17, 41	80, 128	17, 52
Week 104						
n	1	1	2	2	3	3
Mean (SD)	109.0 (NA)	31.0 (NA)	109.5 (7.78)	25.5 (9.19)	109.3 (5.51)	27.3 (7.23)
Median	109.0	31.0	109.5	25.5	109.0	31.0
Min, Max	109, 109	31, 31	104, 115	19, 32	104, 115	19, 32

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.9
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Race
Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	72.5 (9.71)		74.9 (10.83)		73.3 (9.96)	
Median	74.0		75.0		74.5	
Min, Max	55, 91		54, 91		54, 91	
Week 1						
n	21	21	10	10		
Mean (SD)	94.3 (12.01)	22.0 (10.38)	83.0 (11.85)	8.1 (6.24)		
Median	93.0	19.0	81.0	8.5		
Min, Max	70, 116	2, 45	62, 100	-5, 17		
Week 2						
n	20	20	9	9		
Mean (SD)	101.1 (10.60)	28.9 (10.37)	85.2 (8.32)	10.9 (8.55)		
Median	101.0	25.5	83.0	8.0		
Min, Max	80, 122	19, 51	74, 99	3, 32		

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Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.2.9
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Race
Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	18	18	9	9		
Mean (SD)	103.4 (11.60)	32.5 (10.51)	81.1 (10.74)	6.8 (11.18)		
Median	103.5	31.5	82.0	6.0		
Min, Max	83, 122	15, 55	64, 98	-3, 34		
Week 4						
n	19	19	9	9		
Mean (SD)	106.2 (15.60)	34.7 (12.87)	81.3 (9.15)	7.0 (10.75)		
Median	107.0	37.0	85.0	6.0		
Min, Max	74, 126	10, 56	67, 92	-7, 25		
Week 6						
n	22	22	9	9		
Mean (SD)	105.4 (16.86)	32.9 (15.04)	82.7 (10.22)	8.3 (7.66)		
Median	106.5	33.5	86.0	8.0		
Min, Max	73, 133	2, 59	70, 99	-1, 19		

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Table GERMANY.2.6.2.9

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Race
Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	19	19	10	10		
Mean (SD)	106.9 (13.98)	34.9 (14.59)	83.5 (9.14)	8.6 (6.82)		
Median	106.0	33.0	82.0	9.0		
Min, Max	80, 128	4, 68	66, 96	1, 21		
Week 10						
n	17	17	9	9		
Mean (SD)	107.9 (14.36)	35.0 (11.74)	78.8 (15.75)	1.6 (9.42)		
Median	113.0	33.0	82.0	3.0		
Min, Max	80, 128	9, 54	51, 106	-20, 15		
Week 12						
n	21	21	10	10		
Mean (SD)	107.4 (17.36)	34.8 (15.97)	85.6 (8.81)	10.7 (9.88)		
Median	113.0	35.0	86.0	10.5		
Min, Max	68, 131	-8, 60	69, 99	-5, 31		

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Table GERMANY.2.6.2.9
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Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	18	18	8	8		
Mean (SD)	107.6 (18.35)	35.1 (15.40)	94.9 (12.08)	17.3 (8.35)		
Median	113.0	33.0	93.5	15.5		
Min, Max	70, 135	-1, 59	75, 112	4, 31		
Week 14						
n	20	20	8	8		
Mean (SD)	107.6 (20.77)	34.8 (16.99)	107.1 (21.93)	29.5 (18.30)		
Median	112.0	32.5	105.5	26.0		
Min, Max	70, 146	3, 65	81, 152	10, 72		
Week 15						
n	20	20	7	7		
Mean (SD)	109.5 (18.94)	36.0 (16.27)	104.3 (13.52)	27.4 (6.80)		
Median	115.0	34.0	109.0	30.0		
Min, Max	75, 145	2, 64	85, 121	14, 33		

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Table GERMANY.2.6.2.9
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and Long Term Extension by Race
Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	21	21	9	9		
Mean (SD)	105.6 (20.59)	32.5 (17.30)	104.4 (15.73)	29.4 (14.84)		
Median	108.0	30.0	106.0	29.0		
Min, Max	67, 147	-4, 66	74, 122	3, 60		
Week 18						
n	18	18	8	8		
Mean (SD)	104.6 (21.92)	31.4 (19.98)	102.0 (19.15)	24.4 (12.57)		
Median	111.0	30.0	107.5	29.5		
Min, Max	69, 143	-4, 63	73, 124	2, 36		
Week 20						
n	21	21	8	8		
Mean (SD)	107.9 (20.53)	34.8 (17.35)	101.8 (22.07)	24.1 (17.19)		
Median	115.0	33.0	106.5	27.0		
Min, Max	67, 145	-4, 64	66, 125	-5, 49		

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Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	18	18	9	9		
Mean (SD)	110.3 (21.89)	38.0 (19.85)	103.9 (17.29)	28.9 (17.06)		
Median	120.0	42.5	107.0	29.0		
Min, Max	65, 146	-6, 66	70, 125	-1, 53		
Week 24						
n	19	19	8	8		
Mean (SD)	113.2 (20.44)	40.9 (17.76)	104.9 (19.98)	29.4 (15.34)		
Median	119.0	44.0	105.5	29.5		
Min, Max	70, 152	-6, 71	76, 129	0, 51		
Week 28						
n	17	17	8	8	25	25
Mean (SD)	108.4 (22.51)	36.2 (20.96)	99.3 (22.76)	23.8 (19.86)	105.5 (22.54)	32.2 (21.04)
Median	108.0	35.0	101.0	26.5	107.0	32.0
Min, Max	60, 148	-16, 67	64, 129	-12, 45	60, 148	-16, 67

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Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	20	20	7	7	27	27
Mean (SD)	106.1 (18.56)	32.9 (17.95)	101.1 (23.82)	26.3 (18.30)	104.8 (19.69)	31.1 (17.92)
Median	110.0	32.0	96.0	27.0	108.0	31.0
Min, Max	68, 132	-3, 64	66, 132	-2, 48	66, 132	-3, 64
Week 36						
n	18	18	7	7	25	25
Mean (SD)	106.1 (18.04)	33.6 (16.72)	109.3 (13.19)	34.4 (13.38)	107.0 (16.62)	33.8 (15.58)
Median	108.0	32.0	107.0	32.0	107.0	32.0
Min, Max	73, 143	-3, 62	93, 123	10, 50	73, 143	-3, 62
Week 40						
n	18	18	7	7	25	25
Mean (SD)	110.7 (18.78)	37.9 (16.38)	103.0 (24.85)	28.1 (18.73)	108.6 (20.41)	35.2 (17.25)
Median	107.5	34.5	102.0	32.0	107.0	33.0
Min, Max	66, 155	9, 74	57, 131	-5, 47	57, 155	-5, 74

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Modified Randomized Set

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Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	17	17	6	6	23	23
Mean (SD)	106.6 (19.03)	32.9 (14.23)	112.8 (15.99)	39.3 (10.84)	108.2 (18.15)	34.6 (13.50)
Median	109.0	34.0	111.5	39.0	109.0	35.0
Min, Max	65, 146	8, 65	96, 131	26, 55	65, 146	8, 65
Week 48						
n	15	15	6	6	21	21
Mean (SD)	110.4 (19.66)	37.3 (15.97)	107.5 (19.66)	34.0 (10.39)	109.6 (19.21)	36.3 (14.41)
Median	111.0	36.0	114.0	31.5	111.0	34.0
Min, Max	68, 153	11, 72	82, 127	20, 49	68, 153	11, 72
Week 52						
n	13	13	5	5	18	18
Mean (SD)	108.6 (20.60)	35.1 (15.61)	100.4 (27.55)	30.4 (18.99)	106.3 (22.19)	33.8 (16.17)
Median	109.0	38.0	105.0	29.0	108.5	35.0
Min, Max	62, 143	5, 62	64, 127	2, 53	62, 143	2, 62

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Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	15	15	5	5	20	20
Mean (SD)	105.7 (25.40)	32.1 (19.99)	106.2 (20.14)	36.2 (11.48)	105.9 (23.68)	33.1 (18.04)
Median	109.0	33.0	103.0	32.0	108.0	32.5
Min, Max	52, 148	-5, 67	81, 128	27, 54	52, 148	-5, 67
Week 60						
n	13	13	5	5	18	18
Mean (SD)	106.0 (22.21)	31.7 (18.28)	104.8 (19.20)	34.8 (11.39)	105.7 (20.86)	32.6 (16.39)
Median	102.0	32.0	99.0	30.0	102.0	32.0
Min, Max	60, 151	3, 70	84, 126	23, 52	60, 151	3, 70
Week 64						
n	12	12	5	5	17	17
Mean (SD)	105.2 (23.40)	31.7 (19.00)	110.0 (18.64)	40.0 (10.12)	106.6 (21.64)	34.1 (17.01)
Median	104.5	32.0	112.0	38.0	106.0	35.0
Min, Max	47, 137	-10, 56	84, 131	30, 57	47, 137	-10, 57

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	9	9	4	4	13	13
Mean (SD)	100.8 (21.67)	26.8 (16.87)	100.3 (12.12)	31.3 (5.12)	100.6 (18.70)	28.2 (14.17)
Median	106.0	28.0	100.0	30.5	106.0	29.0
Min, Max	50, 129	-7, 48	88, 113	26, 38	50, 129	-7, 48
Week 72						
n	7	7	3	3	10	10
Mean (SD)	99.6 (18.18)	27.7 (13.01)	108.0 (16.52)	34.0 (5.57)	102.1 (17.25)	29.6 (11.35)
Median	97.0	27.0	109.0	33.0	103.0	28.0
Min, Max	68, 119	11, 46	91, 124	29, 40	68, 124	11, 46
Week 80						
n	6	6	3	3	9	9
Mean (SD)	106.0 (15.94)	31.0 (16.21)	107.3 (14.64)	33.3 (5.13)	106.4 (14.59)	31.8 (13.12)
Median	106.5	29.0	105.0	32.0	105.0	32.0
Min, Max	89, 132	13, 59	94, 123	29, 39	89, 132	13, 59

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Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	2	2	1	1	3	3
Mean (SD)	108.5 (27.58)	37.0 (25.46)	98.0 (NA)	22.0 (NA)	105.0 (20.42)	32.0 (19.97)
Median	108.5	37.0	98.0	22.0	98.0	22.0
Min, Max	89, 128	19, 55	98, 98	22, 22	89, 128	19, 55
Week 96						
n	2	2	1	1	3	3
Mean (SD)	121.0 (5.66)	48.0 (5.66)	107.0 (NA)	31.0 (NA)	116.3 (9.02)	42.3 (10.60)
Median	121.0	48.0	107.0	31.0	117.0	44.0
Min, Max	117, 125	44, 52	107, 107	31, 31	107, 125	31, 52

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Source: ADaM.ADSL, ADaM.ADLB

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Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.2.9
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Race
Modified Randomized Set

White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		11		39	
Mean (SD)	79.8 (8.38)		81.1 (11.13)		80.1 (9.10)	
Median	81.0		83.0		81.0	
Min, Max	61, 94		61, 93		61, 94	
Week 1						
n	24	24	9	9		
Mean (SD)	102.0 (10.75)	21.1 (10.31)	88.6 (17.18)	9.0 (12.22)		
Median	100.5	22.0	84.0	10.0		
Min, Max	83, 122	-2, 43	62, 114	-5, 36		
Week 2						
n	28	28	9	9		
Mean (SD)	107.5 (12.53)	27.7 (13.15)	90.4 (18.23)	10.9 (10.51)		
Median	110.0	28.0	92.0	9.0		
Min, Max	84, 133	3, 55	64, 111	-9, 27		

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Table GERMANY.2.6.2.9
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and Long Term Extension by Race
Modified Randomized Set

White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	21	21	8	8		
Mean (SD)	106.9 (13.58)	25.9 (13.57)	89.8 (18.96)	11.8 (8.48)		
Median	107.0	28.0	93.5	11.5		
Min, Max	80, 125	-1, 59	58, 117	-3, 24		
Week 4						
n	27	27	10	10		
Mean (SD)	109.4 (16.62)	29.0 (13.93)	88.6 (18.49)	8.7 (10.69)		
Median	111.0	30.0	91.5	7.5		
Min, Max	74, 139	-7, 58	58, 114	-5, 30		
Week 6						
n	22	22	10	10		
Mean (SD)	108.7 (13.06)	28.4 (10.66)	87.4 (12.26)	7.5 (8.06)		
Median	111.0	28.5	87.0	7.0		
Min, Max	85, 127	8, 47	62, 107	-5, 19		

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and Long Term Extension by Race
Modified Randomized Set

White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	23	23	10	10		
Mean (SD)	102.1 (17.18)	22.4 (14.79)	85.0 (13.26)	5.1 (8.44)		
Median	103.0	20.0	85.0	6.0		
Min, Max	64, 130	-14, 48	67, 106	-9, 18		
Week 10						
n	16	16	9	9		
Mean (SD)	105.4 (14.12)	23.3 (11.36)	88.2 (12.61)	8.1 (8.45)		
Median	101.0	22.5	88.0	11.0		
Min, Max	81, 130	9, 47	62, 104	-4, 20		
Week 12						
n	20	20	9	9		
Mean (SD)	104.8 (13.60)	26.1 (12.82)	84.8 (14.08)	3.0 (5.59)		
Median	104.5	24.5	88.0	5.0		
Min, Max	77, 123	3, 58	54, 100	-7, 11		

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and Long Term Extension by Race
Modified Randomized Set

White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	19	19	8	8		
Mean (SD)	107.9 (13.50)	27.8 (12.93)	96.5 (11.16)	19.8 (6.48)		
Median	107.0	24.0	99.0	17.0		
Min, Max	87, 129	10, 58	76, 110	13, 32		
Week 14						
n	20	20	10	10		
Mean (SD)	108.3 (15.19)	28.7 (16.38)	108.1 (15.07)	28.2 (9.67)		
Median	110.0	31.0	109.5	26.5		
Min, Max	70, 131	-5, 69	89, 137	16, 45		
Week 15						
n	17	17	7	7		
Mean (SD)	100.9 (12.15)	21.1 (12.65)	110.3 (16.81)	29.7 (9.96)		
Median	100.0	20.0	110.0	26.0		
Min, Max	80, 124	-6, 41	87, 141	18, 48		

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Modified Randomized Set

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Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	22	22	10	10		
Mean (SD)	106.0 (14.99)	25.3 (14.86)	107.2 (19.39)	27.3 (14.49)		
Median	104.5	24.0	100.0	28.5		
Min, Max	78, 137	2, 53	90, 153	12, 60		
Week 18						
n	18	18	8	8		
Mean (SD)	98.7 (15.70)	19.7 (17.07)	103.4 (15.43)	22.5 (11.55)		
Median	97.5	13.5	98.0	18.5		
Min, Max	78, 122	-4, 50	92, 139	11, 46		
Week 20						
n	16	16	7	7		
Mean (SD)	103.8 (15.93)	21.7 (14.69)	106.3 (14.22)	24.7 (16.39)		
Median	105.0	23.0	104.0	21.0		
Min, Max	76, 129	2, 48	94, 136	2, 48		

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	20	20	6	6		
Mean (SD)	107.4 (17.20)	27.5 (17.93)	114.5 (16.21)	35.8 (11.82)		
Median	104.5	19.0	110.5	36.5		
Min, Max	84, 132	5, 67	96, 143	20, 50		
Week 24						
n	22	22	7	7		
Mean (SD)	101.1 (14.86)	20.9 (15.43)	106.7 (16.43)	27.4 (14.80)		
Median	99.5	19.0	105.0	33.0		
Min, Max	77, 130	-4, 58	90, 136	9, 44		
Week 28						
n	21	21	6	6	27	27
Mean (SD)	104.0 (17.30)	24.6 (15.88)	110.5 (14.20)	31.0 (13.91)	105.5 (16.62)	26.0 (15.45)
Median	101.0	20.0	106.0	30.5	101.0	20.0
Min, Max	67, 142	0, 58	97, 131	16, 50	67, 142	0, 58

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Modified Randomized Set

White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	19	19	7	7	26	26
Mean (SD)	104.1 (19.02)	24.5 (20.79)	101.1 (11.05)	22.0 (14.17)	103.3 (17.08)	23.8 (18.99)
Median	102.0	23.0	105.0	15.0	103.5	23.0
Min, Max	66, 133	-9, 71	86, 118	8, 45	66, 133	-9, 71
Week 36						
n	13	13	9	9	22	22
Mean (SD)	107.0 (17.22)	28.8 (17.09)	108.8 (14.64)	30.2 (18.47)	107.7 (15.87)	29.4 (17.24)
Median	111.0	28.0	116.0	27.0	112.5	27.5
Min, Max	82, 130	-1, 58	89, 124	8, 61	82, 130	-1, 61
Week 40						
n	15	15	6	6	21	21
Mean (SD)	102.5 (19.03)	25.7 (21.76)	107.2 (15.92)	27.7 (16.90)	103.8 (17.93)	26.3 (20.09)
Median	99.0	21.0	105.5	24.0	99.0	21.0
Min, Max	76, 137	-1, 65	89, 127	13, 53	76, 137	-1, 65

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Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	15	15	8	8	23	23
Mean (SD)	107.1 (23.49)	29.9 (23.46)	105.6 (13.20)	28.4 (15.51)	106.6 (20.18)	29.4 (20.67)
Median	108.0	28.0	102.0	26.5	105.0	28.0
Min, Max	58, 145	-17, 73	89, 125	11, 53	58, 145	-17, 73
Week 48						
n	11	11	7	7	18	18
Mean (SD)	102.7 (18.34)	23.6 (19.21)	109.7 (12.54)	32.3 (12.59)	105.4 (16.30)	27.0 (17.08)
Median	99.0	19.0	111.0	33.0	105.5	29.0
Min, Max	79, 140	6, 68	89, 129	8, 50	79, 140	6, 68
Week 52						
n	11	11	7	7	18	18
Mean (SD)	108.4 (18.06)	29.0 (14.06)	110.3 (19.09)	30.9 (15.74)	109.1 (17.93)	29.7 (14.31)
Median	108.0	30.0	106.0	34.0	107.0	33.5
Min, Max	85, 136	9, 48	86, 142	5, 49	85, 142	5, 49

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	12	12	6	6	18	18
Mean (SD)	108.2 (14.49)	29.6 (13.99)	101.8 (18.27)	24.7 (19.42)	106.1 (15.60)	27.9 (15.60)
Median	109.0	30.5	103.5	28.0	106.0	30.5
Min, Max	88, 126	10, 52	78, 124	-3, 46	78, 126	-3, 52
Week 60						
n	9	9	6	6	15	15
Mean (SD)	109.0 (17.30)	30.8 (16.80)	98.8 (23.05)	17.5 (16.32)	104.9 (19.68)	25.5 (17.37)
Median	112.0	32.0	93.5	13.0	97.0	22.0
Min, Max	89, 137	7, 65	75, 141	4, 48	75, 141	4, 65
Week 64						
n	6	6	6	6	12	12
Mean (SD)	100.3 (12.71)	24.7 (6.06)	94.8 (17.84)	15.2 (13.70)	97.6 (15.04)	19.9 (11.25)
Median	102.5	23.0	94.5	11.5	98.0	21.5
Min, Max	79, 113	18, 35	72, 116	-2, 33	72, 116	-2, 35

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	8	8	5	5	13	13
Mean (SD)	106.6 (19.67)	31.1 (16.68)	94.2 (21.17)	13.8 (14.79)	101.8 (20.36)	24.5 (17.67)
Median	117.5	33.5	104.0	12.0	107.0	24.0
Min, Max	74, 124	13, 59	66, 116	-3, 33	66, 124	-3, 59
Week 72						
n	6	6	4	4	10	10
Mean (SD)	109.5 (18.47)	33.2 (14.91)	90.3 (22.25)	12.8 (21.88)	101.8 (21.29)	25.0 (19.86)
Median	109.5	39.0	84.5	11.5	100.5	29.5
Min, Max	84, 135	8, 47	70, 122	-11, 39	70, 135	-11, 47
Week 80						
n	3	3	4	4	7	7
Mean (SD)	102.7 (25.72)	26.0 (22.34)	102.8 (23.96)	25.3 (14.38)	102.7 (22.53)	25.6 (16.43)
Median	92.0	19.0	113.0	31.0	112.0	29.0
Min, Max	84, 132	8, 51	67, 118	4, 35	67, 132	4, 51

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.2.9
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Race
Modified Randomized Set

White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	3	3	9	9
Mean (SD)	100.5 (20.31)	22.3 (11.06)	93.0 (17.00)	17.3 (8.39)	98.0 (18.55)	20.7 (10.01)
Median	105.0	23.0	93.0	13.0	95.0	19.0
Min, Max	72, 122	10, 35	76, 110	12, 27	72, 122	10, 35
Week 96						
n	2	2	3	3	5	5
Mean (SD)	109.5 (26.16)	32.5 (20.51)	105.0 (22.11)	29.3 (12.01)	106.8 (20.54)	30.6 (13.43)
Median	109.5	32.5	113.0	30.0	113.0	30.0
Min, Max	91, 128	18, 47	80, 122	17, 41	80, 128	17, 47
Week 104						
n	0	0	1	1	1	1
Mean (SD)			115.0 (NA)	32.0 (NA)	115.0 (NA)	32.0 (NA)
Median			115.0	32.0	115.0	32.0
Min, Max			115, 115	32, 32	115, 115	32, 32

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.2.9
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Race
Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		5		12	
Mean (SD)	78.0 (9.02)		82.4 (6.02)		79.8 (7.92)	
Median	79.0		85.0		79.5	
Min, Max	66, 91		76, 89		66, 91	
Week 1						
n	5	5	4	4		
Mean (SD)	100.2 (11.41)	20.2 (11.97)	99.8 (17.15)	15.8 (13.65)		
Median	104.0	19.0	99.5	12.5		
Min, Max	84, 110	2, 32	79, 121	3, 35		
Week 2						
n	7	7	5	5		
Mean (SD)	103.4 (11.79)	25.4 (15.39)	87.4 (12.28)	5.0 (8.51)		
Median	99.0	20.0	86.0	5.0		
Min, Max	86, 119	8, 52	70, 103	-6, 17		

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Table GERMANY.2.6.2.9
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Race
Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	7	7	5	5		
Mean (SD)	107.0 (16.63)	29.0 (18.40)	96.8 (12.79)	14.4 (9.86)		
Median	104.0	18.0	92.0	13.0		
Min, Max	82, 126	13, 58	84, 116	6, 31		
Week 4						
n	6	6	5	5		
Mean (SD)	104.7 (15.50)	24.8 (12.51)	92.6 (9.45)	10.2 (6.61)		
Median	106.5	18.5	91.0	11.0		
Min, Max	79, 122	13, 44	79, 103	3, 18		
Week 6						
n	6	6	5	5		
Mean (SD)	105.0 (14.59)	25.2 (13.36)	91.8 (7.79)	9.4 (4.04)		
Median	104.5	22.0	92.0	8.0		
Min, Max	82, 122	9, 44	81, 101	5, 15		

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Table GERMANY.2.6.2.9
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and Long Term Extension by Race
Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	4	4	4	4		
Mean (SD)	96.8 (17.06)	18.0 (17.47)	101.5 (9.47)	17.5 (5.92)		
Median	91.5	12.0	102.5	15.5		
Min, Max	83, 121	5, 43	89, 112	13, 26		
Week 10						
n	5	5	4	4		
Mean (SD)	107.2 (16.07)	29.8 (18.51)	90.8 (11.53)	6.8 (10.84)		
Median	111.0	26.0	90.0	7.5		
Min, Max	83, 121	9, 54	78, 105	-7, 19		
Week 12						
n	6	6	5	5		
Mean (SD)	104.5 (12.77)	26.7 (15.34)	89.8 (10.03)	7.4 (8.68)		
Median	107.0	24.5	87.0	3.0		
Min, Max	86, 118	9, 51	79, 106	0, 21		

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Table GERMANY.2.6.2.9
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and Long Term Extension by Race
Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	5	5	5	5		
Mean (SD)	96.2 (12.56)	17.4 (12.22)	99.6 (7.50)	17.2 (10.55)		
Median	95.0	14.0	102.0	21.0		
Min, Max	80, 115	4, 37	87, 106	1, 28		
Week 14						
n	5	5	4	4		
Mean (SD)	95.8 (15.14)	17.0 (14.05)	103.5 (10.25)	19.5 (14.57)		
Median	94.0	11.0	105.0	18.0		
Min, Max	76, 117	3, 39	90, 114	4, 38		
Week 15						
n	3	3	5	5		
Mean (SD)	105.7 (7.64)	23.0 (11.79)	106.4 (13.76)	24.0 (16.55)		
Median	104.0	20.0	104.0	19.0		
Min, Max	99, 114	13, 36	93, 126	7, 50		

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Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	3	3	5	5		
Mean (SD)	110.0 (10.44)	25.3 (16.50)	102.6 (13.87)	20.2 (15.55)		
Median	115.0	30.0	103.0	14.0		
Min, Max	98, 117	7, 39	87, 120	5, 44		
Week 18						
n	4	4	4	4		
Mean (SD)	98.0 (10.00)	19.5 (10.47)	104.5 (19.21)	23.0 (17.38)		
Median	97.0	18.5	112.0	25.0		
Min, Max	87, 111	8, 33	76, 118	0, 42		
Week 20						
n	4	4	4	4		
Mean (SD)	99.0 (16.06)	20.3 (14.52)	94.3 (7.09)	10.3 (2.63)		
Median	102.5	22.0	97.0	9.5		
Min, Max	78, 113	4, 33	84, 99	8, 14		

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and Long Term Extension by Race
Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	5	5	4	4		
Mean (SD)	95.2 (16.72)	15.4 (14.06)	97.3 (13.48)	13.3 (10.78)		
Median	95.0	8.0	97.0	9.5		
Min, Max	74, 114	4, 36	81, 114	5, 29		
Week 24						
n	6	6	3	3		
Mean (SD)	100.3 (16.43)	20.5 (11.41)	97.7 (16.65)	14.3 (11.50)		
Median	106.5	22.0	103.0	14.0		
Min, Max	70, 114	4, 31	79, 111	3, 26		
Week 28						
n	4	4	4	4	8	8
Mean (SD)	94.8 (14.22)	19.0 (13.71)	98.5 (4.80)	14.5 (5.80)	96.6 (10.03)	16.8 (10.04)
Median	88.5	15.0	99.0	15.5	94.5	15.5
Min, Max	86, 116	8, 38	93, 103	7, 20	86, 116	7, 38

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Table GERMANY.2.6.2.9

Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Race
Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	5	5	4	4	9	9
Mean (SD)	101.2 (9.52)	22.4 (9.89)	101.8 (4.27)	17.8 (6.40)	101.4 (7.23)	20.3 (8.38)
Median	102.0	25.0	100.0	19.0	101.0	23.0
Min, Max	91, 111	11, 32	99, 108	10, 23	91, 111	10, 32
Week 36						
n	4	4	4	4	8	8
Mean (SD)	108.3 (21.11)	27.8 (21.09)	102.8 (12.15)	18.8 (8.30)	105.5 (16.21)	23.3 (15.60)
Median	115.0	33.5	105.0	17.5	108.0	21.5
Min, Max	78, 125	-1, 45	86, 115	10, 30	78, 125	-1, 45
Week 40						
n	4	4	5	5	9	9
Mean (SD)	101.5 (16.70)	19.5 (17.25)	100.4 (5.13)	18.0 (8.03)	100.9 (10.87)	18.7 (12.02)
Median	105.5	21.0	98.0	22.0	101.0	22.0
Min, Max	78, 117	-1, 37	96, 109	7, 25	78, 117	-1, 37

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Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	5	5	2	2	7	7
Mean (SD)	104.6 (12.62)	22.0 (12.31)	112.5 (10.61)	25.5 (13.44)	106.9 (11.82)	23.0 (11.58)
Median	107.0	27.0	112.5	25.5	107.0	27.0
Min, Max	83, 115	4, 34	105, 120	16, 35	83, 120	4, 35
Week 48						
n	5	5	5	5	10	10
Mean (SD)	97.8 (13.75)	18.0 (14.70)	101.4 (12.01)	19.0 (13.71)	99.6 (12.32)	18.5 (13.41)
Median	94.0	13.0	101.0	13.0	97.5	13.0
Min, Max	82, 118	3, 40	89, 114	4, 38	82, 118	3, 40
Week 52						
n	3	3	4	4	7	7
Mean (SD)	99.0 (20.22)	24.7 (24.09)	105.0 (10.10)	21.0 (10.13)	102.4 (14.06)	22.6 (15.77)
Median	107.0	36.0	107.5	25.0	107.0	26.0
Min, Max	76, 114	-3, 41	92, 113	6, 28	76, 114	-3, 41

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Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	5	5	5	5	10	10
Mean (SD)	97.8 (19.94)	19.0 (19.16)	107.2 (9.73)	24.8 (9.88)	102.5 (15.60)	21.9 (14.69)
Median	93.0	12.0	111.0	26.0	107.0	21.5
Min, Max	78, 127	2, 47	91, 116	15, 40	78, 127	2, 47
Week 60						
n	2	2	4	4	6	6
Mean (SD)	96.0 (26.87)	17.5 (27.58)	102.3 (8.54)	18.3 (12.28)	100.2 (14.09)	18.0 (15.58)
Median	96.0	17.5	105.0	18.0	105.0	18.0
Min, Max	77, 115	-2, 37	90, 109	4, 33	77, 115	-2, 37
Week 64						
n	3	3	3	3	6	6
Mean (SD)	110.3 (9.45)	31.3 (10.41)	104.3 (8.02)	22.0 (13.11)	107.3 (8.50)	26.7 (11.76)
Median	107.0	28.0	105.0	20.0	106.0	25.5
Min, Max	103, 121	23, 43	96, 112	10, 36	96, 121	10, 43

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Run Date: 2024-04-26T15:49:51

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.2.9
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Race
Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	2	2	3	3	5	5
Mean (SD)	113.5 (0.71)	35.0 (1.41)	110.3 (20.21)	28.0 (25.53)	111.6 (14.40)	30.8 (18.47)
Median	113.5	35.0	107.0	22.0	113.0	34.0
Min, Max	113, 114	34, 36	92, 132	6, 56	92, 132	6, 56
Week 72						
n	3	3	2	2	5	5
Mean (SD)	107.3 (1.53)	28.3 (1.53)	100.0 (12.73)	14.5 (13.44)	104.4 (7.60)	22.8 (10.18)
Median	107.0	28.0	100.0	14.5	107.0	27.0
Min, Max	106, 109	27, 30	91, 109	5, 24	91, 109	5, 30
Week 80						
n	2	2	0	0	2	2
Mean (SD)	91.5 (6.36)	12.0 (5.66)			91.5 (6.36)	12.0 (5.66)
Median	91.5	12.0			91.5	12.0
Min, Max	87, 96	8, 16			87, 96	8, 16

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:49:51

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.2.9
Summary of Actual Values and Change from Baseline in Hemoglobin (g/L) During Treatment Period 1 and Period 2
and Long Term Extension by Race
Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	1	1	2	2	3	3
Mean (SD)	113.0 (NA)	35.0 (NA)	107.0 (4.24)	26.5 (10.61)	109.0 (4.58)	29.3 (8.96)
Median	113.0	35.0	107.0	26.5	110.0	34.0
Min, Max	113, 113	35, 35	104, 110	19, 34	104, 113	19, 35
Week 96						
n	1	1	0	0	1	1
Mean (SD)	101.0 (NA)	23.0 (NA)			101.0 (NA)	23.0 (NA)
Median	101.0	23.0			101.0	23.0
Min, Max	101, 101	23, 23			101, 101	23, 23
Week 104						
n	1	1	1	1	2	2
Mean (SD)	109.0 (NA)	31.0 (NA)	104.0 (NA)	19.0 (NA)	106.5 (3.54)	25.0 (8.49)
Median	109.0	31.0	104.0	19.0	106.5	25.0
Min, Max	109, 109	31, 31	104, 104	19, 19	104, 109	19, 31

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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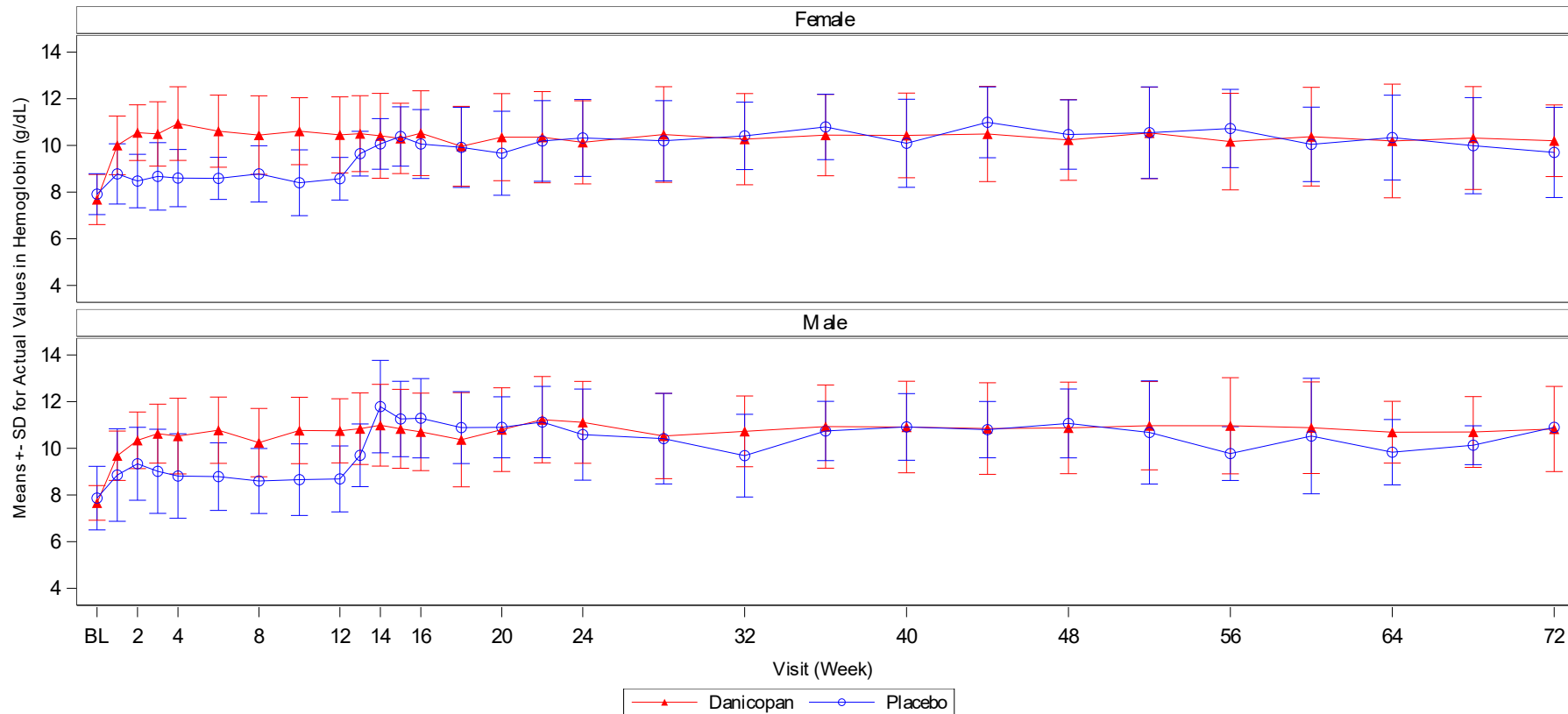
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Figure GERMANY.2.1.1.4.2
Plots of Mean \pm SD for Actual Values in Hemoglobin (g/dL) During 72 Weeks by Sex
Modified Randomized Set



Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Source: ADaM.ADLB

Run Date: 2024-04-26T15:31:00

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Data cutoff date of 31Mar2023

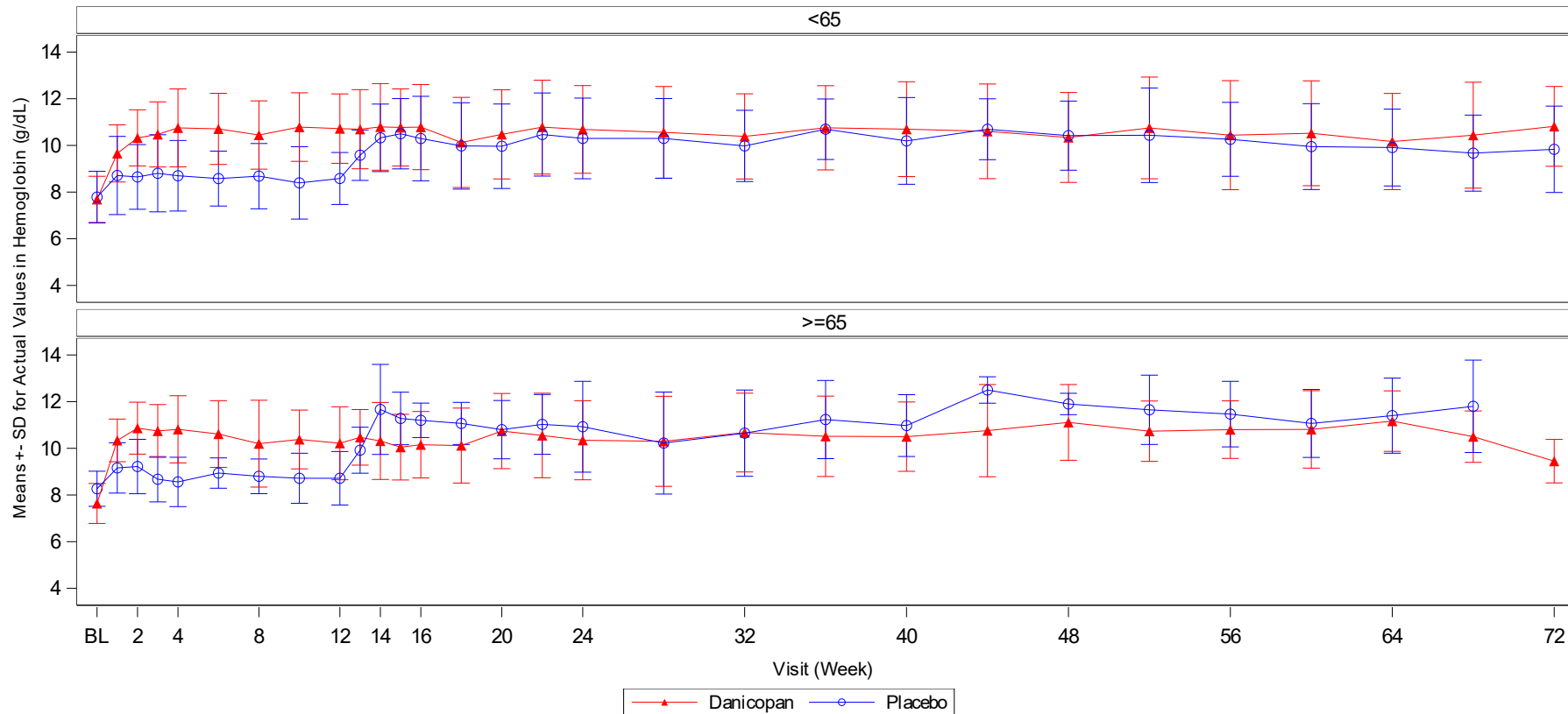
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Figure GERMANY.2.1.1.4.3

Plots of Mean \pm SD for Actual Values in Hemoglobin (g/dL) During 72 Weeks by Age at Informed Consent (< 65 and \geq 65 Years)
Modified Randomized Set



Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Source: ADaM.ADLB

Run Date: 2024-04-26T15:31:01

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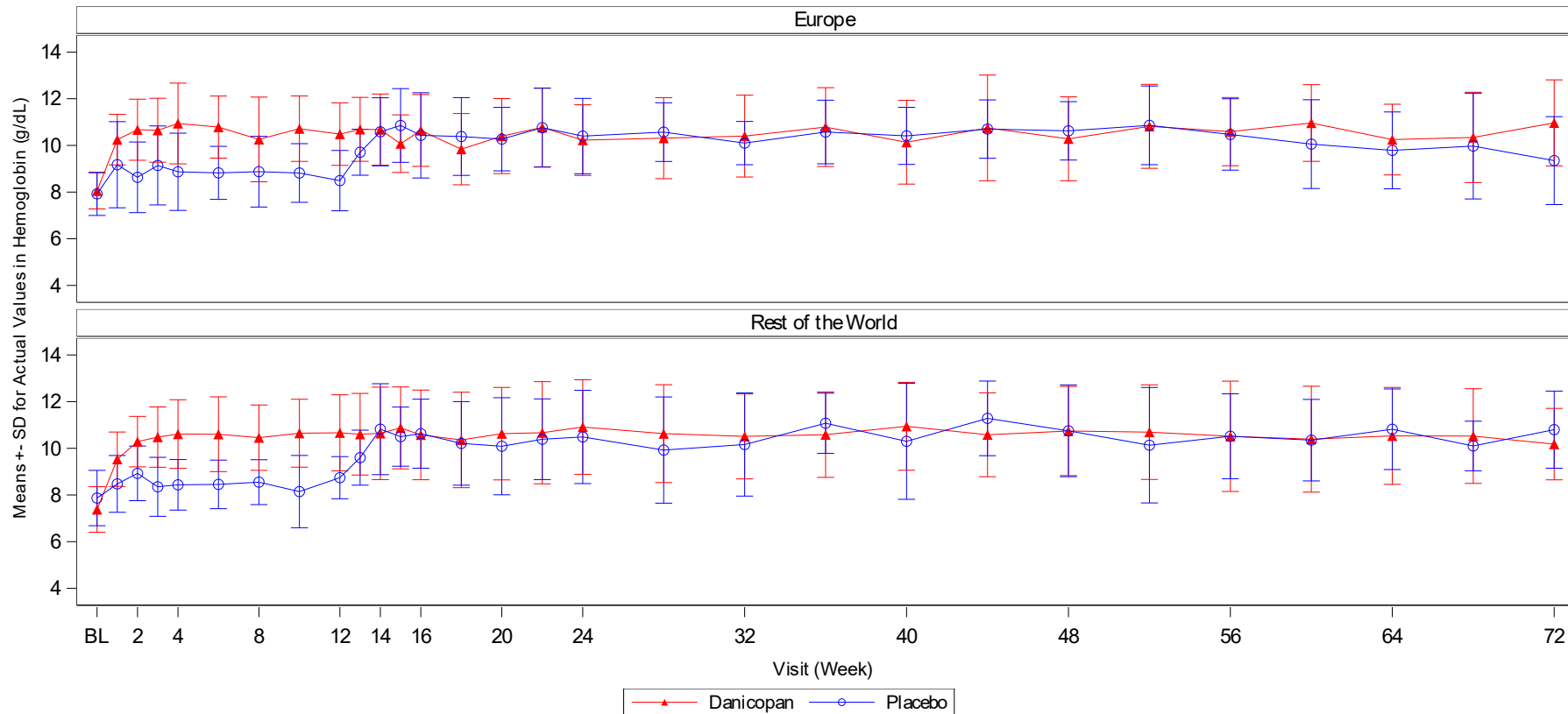
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Figure GERMANY.2.1.1.4.4
Plots of Mean \pm SD for Actual Values in Hemoglobin (g/dL) During 72 Weeks by Region
Modified Randomized Set



Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Source: ADaM.ADLB

Run Date: 2024-04-26T15:31:01

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Data cutoff date of 31Mar2023

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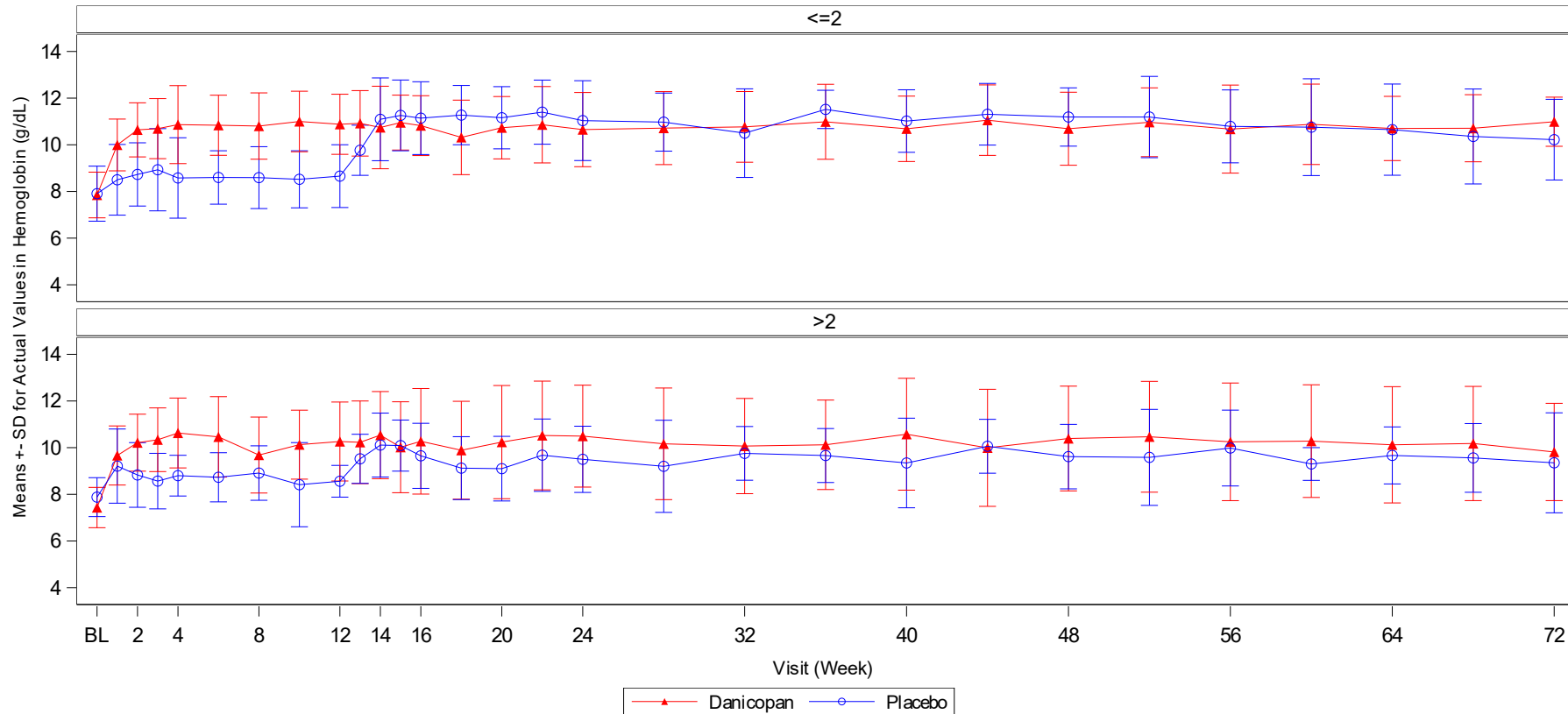
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Figure GERMANY.2.1.1.4.5

Plots of Mean \pm SD for Actual Values in Hemoglobin (g/dL) During 72 Weeks by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set



Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Source: ADaM.ADLB

Run Date: 2024-04-26T15:31:02

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Data cutoff date of 31Mar2023

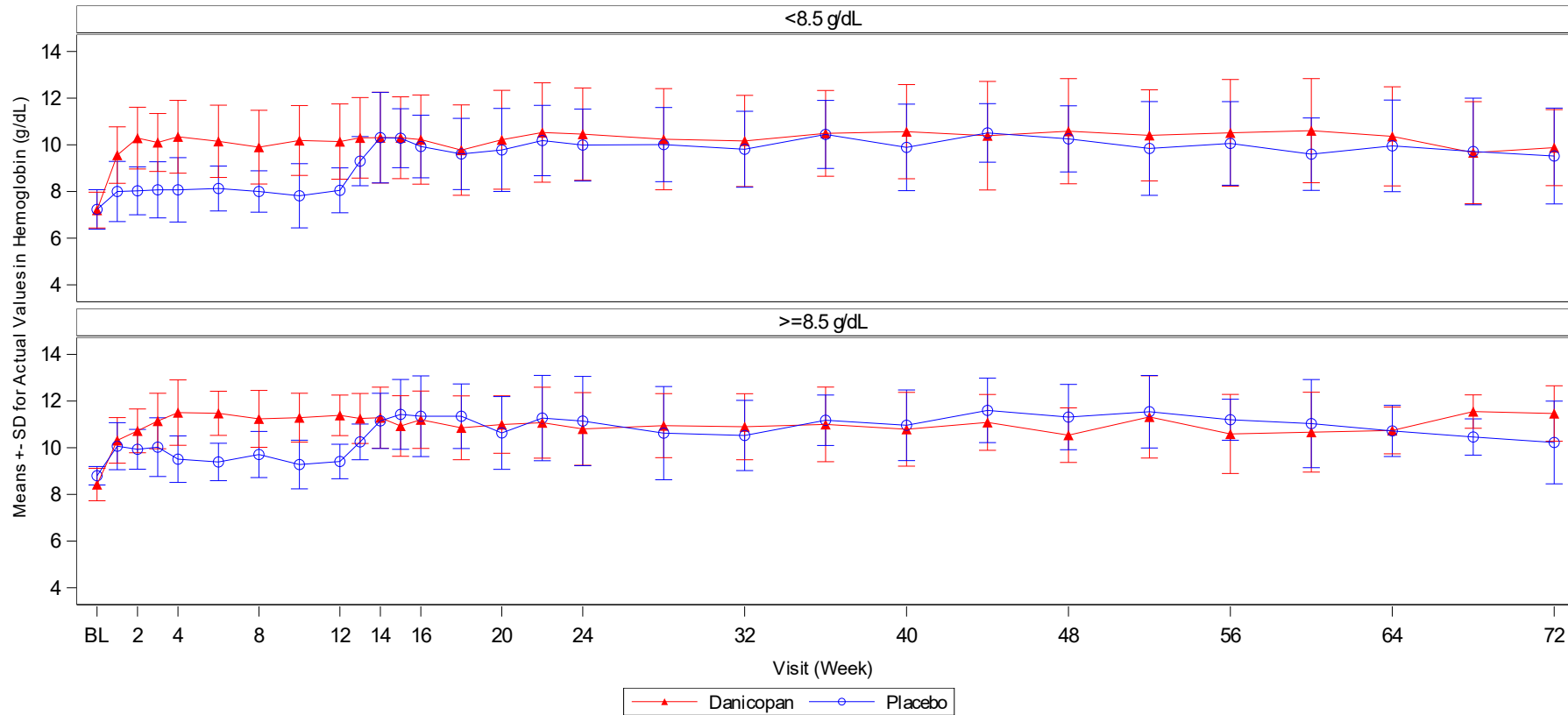
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Figure GERMANY.2.1.1.4.6
Plots of Mean \pm SD for Actual Values in Hemoglobin (g/dL) During 72 Weeks by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set



Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Source: ADaM.ADLB

Run Date: 2024-04-26T15:31:02

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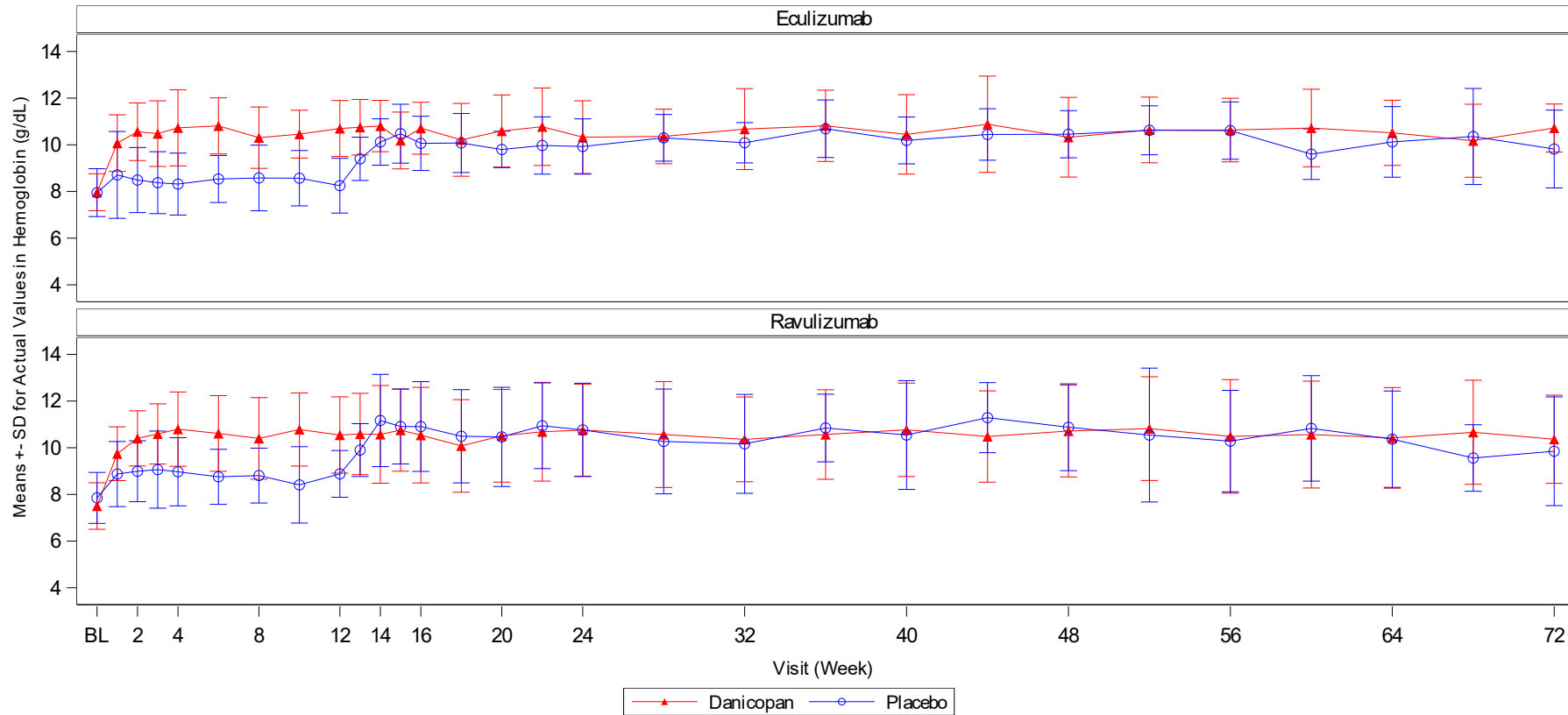
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Figure GERMANY.2.1.1.4.7
Plots of Mean \pm SD for Actual Values in Hemoglobin (g/dL) During 72 Weeks
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set



Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Source: ADaM.ADLB

Run Date: 2024-04-26T15:31:03

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Data cutoff date of 31Mar2023

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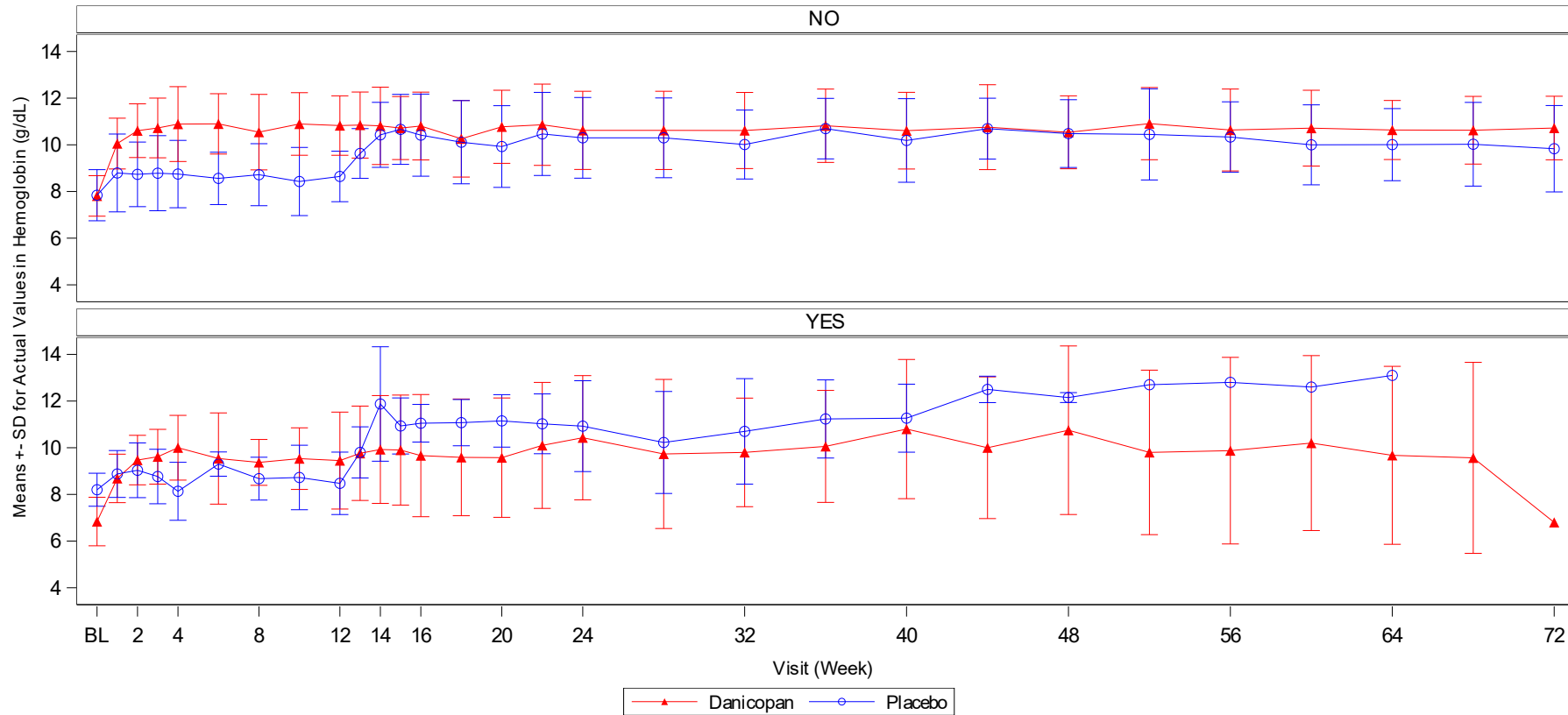
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Figure GERMANY.2.1.1.4.8

Plots of Mean \pm SD for Actual Values in Hemoglobin (g/dL) During 72 Weeks by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set



Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Source: ADaM.ADLB

Run Date: 2024-04-26T15:31:04

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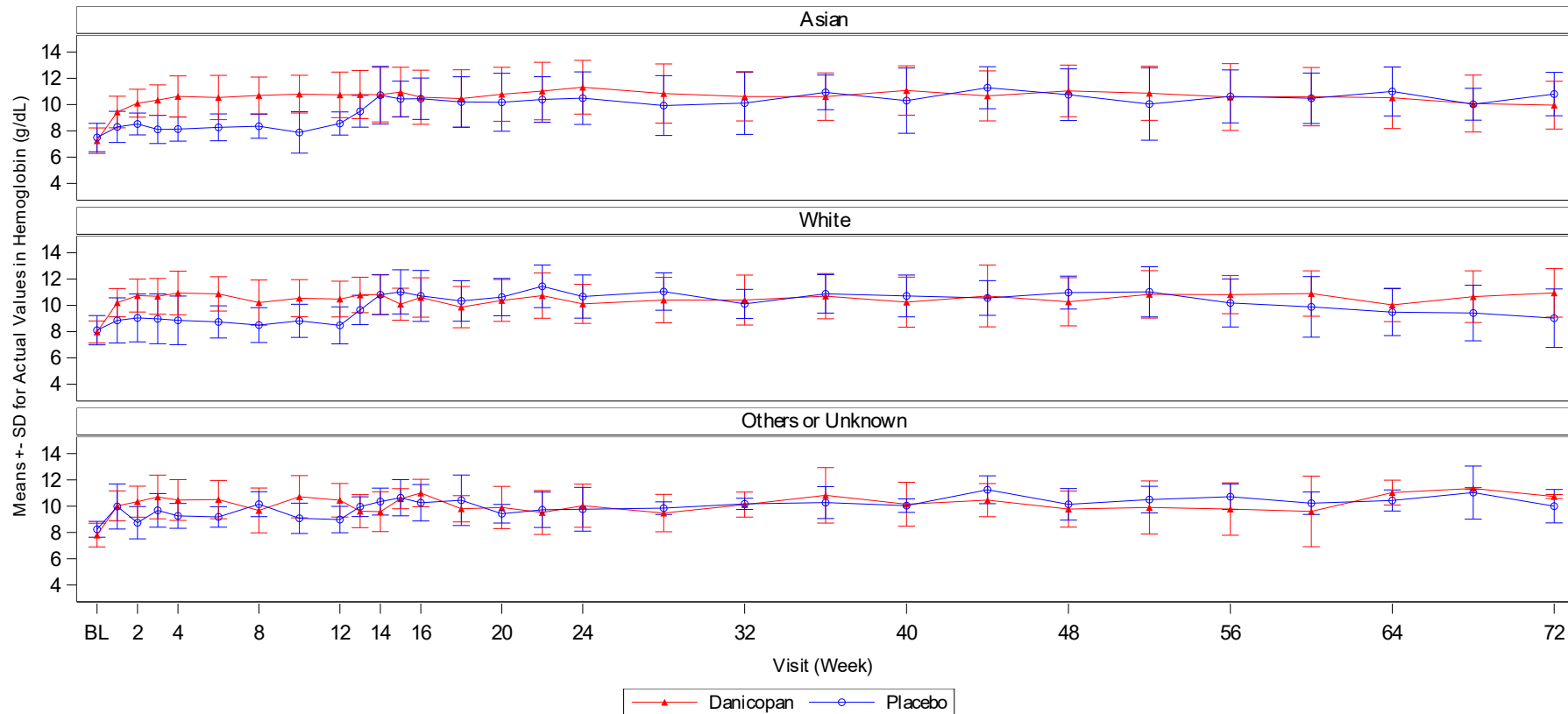
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Figure GERMANY.2.1.1.4.9
Plots of Mean \pm SD for Actual Values in Hemoglobin (g/dL) During 72 Weeks by Race
Modified Randomized Set



Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

Source: ADaM.ADLB

Run Date: 2024-04-26T15:31:04

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Table GERMANY.2.1.1.1.1
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	20.50 (2.339)	10.03 (3.893)	10.47 (4.456)	0.0292
	95% CI for LS MEAN	(15.62, 25.38)	(1.90, 18.16)	(1.17, 19.76)	
Week 2	LS Mean (SEM)	26.63 (2.415)	11.99 (4.197)	14.64 (4.749)	0.0044
	95% CI for LS MEAN	(21.68, 31.57)	(3.43, 20.55)	(4.94, 24.34)	
Week 3	LS Mean (SEM)	29.79 (2.693)	10.39 (4.694)	19.41 (5.316)	0.0011
	95% CI for LS MEAN	(24.24, 35.34)	(0.77, 20.00)	(8.49, 30.32)	
Week 4	LS Mean (SEM)	28.74 (3.369)	9.97 (6.199)	18.77 (6.989)	0.0156
	95% CI for LS MEAN	(21.47, 36.01)	(-3.04, 22.99)	(4.03, 33.51)	
Week 6	LS Mean (SEM)	29.63 (3.079)	5.79 (5.716)	23.84 (6.447)	0.0015
	95% CI for LS MEAN	(23.18, 36.09)	(-6.18, 17.76)	(10.35, 37.34)	
Week 8	LS Mean (SEM)	25.25 (3.740)	4.57 (5.977)	20.68 (6.974)	0.0144
	95% CI for LS MEAN	(16.80, 33.71)	(-8.73, 17.88)	(5.10, 36.26)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

Run Date: 2024-04-26T15:49:14

Data cutoff date of 31Mar2023

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Table GERMANY.2.1.1.1.1
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	24.61 (4.251)	4.46 (7.738)	20.15 (8.737)	0.0362
	95% CI for LS MEAN	(15.51, 33.71)	(-12.03, 20.96)	(1.49, 38.81)	
Week 12	LS Mean (SEM)	28.03 (3.103)	7.18 (5.039)	20.85 (5.808)	0.0021
	95% CI for LS MEAN	(21.51, 34.55)	(-3.40, 17.76)	(8.63, 33.07)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Table GERMANY.2.1.1.1.1
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 16)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	21.59 (1.892)	8.32 (2.609)	13.27 (3.201)	0.0002
	95% CI for LS MEAN	(17.77, 25.42)	(3.04, 13.60)	(6.80, 19.75)	
Week 2	LS Mean (SEM)	27.87 (2.012)	3.87 (2.916)	24.00 (3.517)	<0.0001
	95% CI for LS MEAN	(23.80, 31.95)	(-2.03, 9.77)	(16.88, 31.12)	
Week 3	LS Mean (SEM)	28.56 (2.246)	3.80 (3.406)	24.76 (4.056)	<0.0001
	95% CI for LS MEAN	(24.01, 33.10)	(-3.07, 10.67)	(16.57, 32.95)	
Week 4	LS Mean (SEM)	32.41 (2.597)	1.34 (4.134)	31.07 (4.871)	<0.0001
	95% CI for LS MEAN	(27.15, 37.67)	(-6.99, 9.67)	(21.23, 40.91)	
Week 6	LS Mean (SEM)	30.09 (2.501)	2.27 (3.902)	27.81 (4.618)	<0.0001
	95% CI for LS MEAN	(25.00, 35.17)	(-5.63, 10.17)	(18.45, 37.18)	
Week 8	LS Mean (SEM)	29.31 (2.854)	5.68 (4.535)	23.63 (5.337)	<0.0001
	95% CI for LS MEAN	(23.54, 35.07)	(-3.46, 14.82)	(12.86, 34.39)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

Run Date: 2024-04-26T15:49:14

Data cutoff date of 31Mar2023

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Table GERMANY.2.1.1.1.1
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 16)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	28.29 (2.825)	5.04 (4.344)	23.26 (5.159)	<0.0001
	95% CI for LS MEAN	(22.55, 34.03)	(-3.79, 13.86)	(12.77, 33.74)	
Week 12	LS Mean (SEM)	27.23 (2.854)	2.33 (4.348)	24.91 (5.184)	<0.0001
	95% CI for LS MEAN	(21.43, 33.03)	(-6.50, 11.15)	(14.38, 35.43)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:49:14

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Table GERMANY.2.1.1.1.1
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.5048
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.1.1.1.2
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 19)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	18.87 (1.747)	8.43 (2.461)	10.44 (2.988)	0.0011
	95% CI for LS MEAN	(15.35, 22.38)	(3.48, 13.38)	(4.42, 16.45)	
Week 2	LS Mean (SEM)	25.42 (1.847)	5.83 (2.794)	19.59 (3.319)	<0.0001
	95% CI for LS MEAN	(21.71, 29.12)	(0.23, 11.42)	(12.94, 26.24)	
Week 3	LS Mean (SEM)	29.04 (2.231)	5.05 (3.425)	23.99 (4.062)	<0.0001
	95% CI for LS MEAN	(24.56, 33.52)	(-1.82, 11.92)	(15.84, 32.14)	
Week 4	LS Mean (SEM)	30.78 (2.544)	3.69 (4.103)	27.09 (4.805)	<0.0001
	95% CI for LS MEAN	(25.66, 35.90)	(-4.53, 11.92)	(17.45, 36.73)	
Week 6	LS Mean (SEM)	30.26 (2.249)	4.02 (3.480)	26.24 (4.121)	<0.0001
	95% CI for LS MEAN	(25.72, 34.79)	(-2.98, 11.01)	(17.95, 34.54)	
Week 8	LS Mean (SEM)	28.51 (2.247)	4.99 (3.410)	23.52 (4.054)	<0.0001
	95% CI for LS MEAN	(23.99, 33.02)	(-1.86, 11.84)	(15.37, 31.66)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.2
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 19)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	29.26 (2.182)	4.74 (3.322)	24.52 (3.939)	<0.0001
	95% CI for LS MEAN	(24.88, 33.64)	(-1.92, 11.41)	(16.61, 32.43)	
Week 12	LS Mean (SEM)	29.03 (2.323)	4.50 (3.584)	24.54 (4.240)	<0.0001
	95% CI for LS MEAN	(24.36, 33.71)	(-2.70, 11.69)	(16.01, 33.06)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Table GERMANY.2.1.1.1.2
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	24.07 (3.450)	10.33 (5.861)	13.74 (6.874)	0.0550
	95% CI for LS MEAN	(17.04, 31.10)	(-1.65, 22.31)	(-0.32, 27.79)	
Week 2	LS Mean (SEM)	30.18 (3.519)	9.84 (5.900)	20.34 (6.946)	0.0064
	95% CI for LS MEAN	(23.02, 37.34)	(-2.21, 21.89)	(6.16, 34.52)	
Week 3	LS Mean (SEM)	29.94 (3.535)	7.71 (6.226)	22.23 (7.228)	0.0041
	95% CI for LS MEAN	(22.75, 37.12)	(-4.92, 20.34)	(7.54, 36.91)	
Week 4	LS Mean (SEM)	29.51 (3.452)	3.40 (5.949)	26.10 (6.955)	0.0007
	95% CI for LS MEAN	(22.47, 36.54)	(-8.73, 15.54)	(11.91, 40.30)	
Week 6	LS Mean (SEM)	31.52 (3.474)	-1.09 (6.437)	32.61 (7.388)	<0.0001
	95% CI for LS MEAN	(24.44, 38.59)	(-14.11, 11.94)	(17.62, 47.59)	
Week 8	LS Mean (SEM)	28.16 (3.535)	3.60 (7.522)	24.57 (8.382)	0.0051
	95% CI for LS MEAN	(20.98, 35.35)	(-11.48, 18.68)	(7.72, 41.41)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.2
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	26.52 (3.793)	5.59 (6.608)	20.93 (7.686)	0.0095
	95% CI for LS MEAN	(18.85, 34.18)	(-7.74, 18.92)	(5.40, 36.45)	
Week 12	LS Mean (SEM)	26.70 (3.694)	4.86 (6.229)	21.84 (7.335)	0.0052
	95% CI for LS MEAN	(19.22, 34.18)	(-7.78, 17.50)	(6.95, 36.73)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Table GERMANY.2.1.1.1.2

Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.4778
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.3
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 13)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	20.86 (2.359)	10.73 (3.451)	10.13 (4.178)	0.0209
	95% CI for LS MEAN	(16.05, 25.67)	(3.71, 17.75)	(1.63, 18.63)	
Week 2	LS Mean (SEM)	25.88 (2.768)	7.11 (3.967)	18.76 (4.820)	0.0005
	95% CI for LS MEAN	(20.23, 31.52)	(-0.97, 15.20)	(8.94, 28.59)	
Week 3	LS Mean (SEM)	26.90 (2.701)	8.18 (4.002)	18.72 (4.803)	0.0005
	95% CI for LS MEAN	(21.38, 32.42)	(0.04, 16.33)	(8.93, 28.50)	
Week 4	LS Mean (SEM)	28.30 (3.276)	7.40 (5.374)	20.90 (6.271)	0.0024
	95% CI for LS MEAN	(21.56, 35.04)	(-3.59, 18.38)	(8.07, 33.74)	
Week 6	LS Mean (SEM)	29.29 (2.737)	5.40 (4.016)	23.88 (4.824)	<0.0001
	95% CI for LS MEAN	(23.59, 34.99)	(-2.92, 13.72)	(13.87, 33.89)	
Week 8	LS Mean (SEM)	24.87 (3.891)	10.16 (5.857)	14.71 (6.970)	0.0445
	95% CI for LS MEAN	(16.85, 32.88)	(-1.86, 22.17)	(0.39, 29.03)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.3
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 13)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	27.07 (3.379)	7.45 (5.139)	19.61 (6.128)	0.0062
	95% CI for LS MEAN	(19.83, 34.31)	(-3.52, 18.42)	(6.51, 32.72)	
Week 12	LS Mean (SEM)	24.80 (2.743)	3.56 (3.830)	21.23 (4.674)	0.0002
	95% CI for LS MEAN	(19.08, 30.52)	(-4.41, 11.54)	(11.50, 30.97)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.3
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	21.09 (1.862)	8.15 (2.715)	12.94 (3.256)	0.0004
	95% CI for LS MEAN	(17.30, 24.88)	(2.61, 13.69)	(6.30, 19.59)	
Week 2	LS Mean (SEM)	28.42 (1.813)	9.18 (3.110)	19.24 (3.573)	<0.0001
	95% CI for LS MEAN	(24.74, 32.10)	(2.89, 15.48)	(12.00, 26.47)	
Week 3	LS Mean (SEM)	31.35 (2.172)	5.96 (3.745)	25.39 (4.310)	<0.0001
	95% CI for LS MEAN	(26.94, 35.77)	(-1.63, 13.55)	(16.65, 34.13)	
Week 4	LS Mean (SEM)	32.14 (2.342)	3.19 (4.048)	28.95 (4.654)	<0.0001
	95% CI for LS MEAN	(27.37, 36.91)	(-5.02, 11.40)	(19.50, 38.41)	
Week 6	LS Mean (SEM)	32.04 (2.574)	2.06 (4.561)	29.97 (5.217)	<0.0001
	95% CI for LS MEAN	(26.80, 37.28)	(-7.16, 11.29)	(19.40, 40.55)	
Week 8	LS Mean (SEM)	31.13 (2.378)	4.00 (4.105)	27.14 (4.693)	<0.0001
	95% CI for LS MEAN	(26.31, 35.95)	(-4.30, 12.29)	(17.63, 36.64)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.3
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	30.59 (2.711)	3.80 (4.504)	26.79 (5.241)	<0.0001
	95% CI for LS MEAN	(25.09, 36.09)	(-5.33, 12.92)	(16.17, 37.42)	
Week 12	LS Mean (SEM)	30.72 (2.731)	6.51 (4.691)	24.20 (5.412)	<0.0001
	95% CI for LS MEAN	(25.19, 36.25)	(-2.95, 15.98)	(13.27, 35.14)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.3
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.4840
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.4
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	22.29 (2.366)	10.42 (3.662)	11.87 (4.402)	0.0129
	95% CI for LS MEAN	(17.40, 27.19)	(2.84, 18.00)	(2.77, 20.98)	
Week 2	LS Mean (SEM)	26.99 (2.656)	7.09 (4.264)	19.89 (5.057)	0.0006
	95% CI for LS MEAN	(21.50, 32.48)	(-1.69, 15.88)	(9.47, 30.31)	
Week 3	LS Mean (SEM)	28.62 (2.937)	2.20 (4.901)	26.41 (5.744)	<0.0001
	95% CI for LS MEAN	(22.57, 34.66)	(-7.81, 12.22)	(14.66, 38.17)	
Week 4	LS Mean (SEM)	31.08 (3.713)	0.12 (6.206)	30.95 (7.252)	0.0003
	95% CI for LS MEAN	(23.31, 38.85)	(-12.75, 13.00)	(15.88, 46.03)	
Week 6	LS Mean (SEM)	29.65 (3.465)	0.84 (6.140)	28.80 (7.076)	0.0004
	95% CI for LS MEAN	(22.49, 36.80)	(-11.71, 13.40)	(14.30, 43.30)	
Week 8	LS Mean (SEM)	26.12 (4.843)	-3.80 (8.725)	29.92 (9.973)	0.0065
	95% CI for LS MEAN	(15.98, 36.26)	(-21.82, 14.22)	(9.26, 50.57)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.
The difference of LSM is calculated as the Danicopan mean minus Placebo mean.
Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value.
P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.
Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.
Source: ADaM.ADSL, ADaM.ADEFF
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Table GERMANY.2.1.1.1.4
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	24.99 (3.654)	1.79 (6.677)	23.20 (7.656)	0.0094
	95% CI for LS MEAN	(17.06, 32.93)	(-12.57, 16.15)	(6.70, 39.70)	
Week 12	LS Mean (SEM)	25.49 (3.421)	-3.47 (5.954)	28.96 (6.897)	0.0002
	95% CI for LS MEAN	(18.47, 32.51)	(-15.58, 8.63)	(14.92, 43.01)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.
The difference of LSM is calculated as the Danicopan mean minus Placebo mean.
Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value.
P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.
Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.
Source: ADaM.ADSL, ADaM.ADEFF
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Table GERMANY.2.1.1.1.4
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	20.09 (2.185)	6.15 (3.199)	13.94 (3.869)	0.0005
	95% CI for LS MEAN	(15.75, 24.42)	(-0.21, 12.50)	(6.26, 21.62)	
Week 2	LS Mean (SEM)	27.35 (2.151)	5.92 (3.307)	21.43 (3.939)	<0.0001
	95% CI for LS MEAN	(23.08, 31.62)	(-0.64, 12.48)	(13.62, 29.24)	
Week 3	LS Mean (SEM)	29.77 (2.199)	6.10 (3.418)	23.66 (4.059)	<0.0001
	95% CI for LS MEAN	(25.40, 34.13)	(-0.67, 12.88)	(15.62, 31.71)	
Week 4	LS Mean (SEM)	29.86 (2.163)	5.16 (3.483)	24.70 (4.094)	<0.0001
	95% CI for LS MEAN	(25.57, 34.16)	(-1.73, 12.06)	(16.59, 32.81)	
Week 6	LS Mean (SEM)	31.08 (2.168)	4.35 (3.385)	26.73 (4.014)	<0.0001
	95% CI for LS MEAN	(26.78, 35.39)	(-2.36, 11.06)	(18.77, 34.69)	
Week 8	LS Mean (SEM)	29.65 (2.212)	5.44 (3.440)	24.21 (4.085)	<0.0001
	95% CI for LS MEAN	(25.26, 34.04)	(-1.37, 12.26)	(16.11, 32.31)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.
The difference of LSM is calculated as the Danicopan mean minus Placebo mean.
Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value.
P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.
Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.
Source: ADaM.ADSL, ADaM.ADEFF
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Table GERMANY.2.1.1.1.4
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	31.49 (2.237)	5.85 (3.347)	25.64 (4.019)	<0.0001
	95% CI for LS MEAN	(27.05, 35.92)	(-0.79, 12.48)	(17.67, 33.61)	
Week 12	LS Mean (SEM)	31.30 (2.209)	6.55 (3.231)	24.74 (3.904)	<0.0001
	95% CI for LS MEAN	(26.92, 35.68)	(0.14, 12.97)	(17.00, 32.49)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.
The difference of LSM is calculated as the Danicopan mean minus Placebo mean.
Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value.
P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.
Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.
Source: ADaM.ADSL, ADaM.ADEFF
Run Date: 2024-04-26T15:49:16
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Table GERMANY.2.1.1.1.4
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of
Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.3725
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.5
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of
Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	23.58 (2.170)	7.76 (3.007)	15.82 (3.710)	0.0001
	95% CI for LS MEAN	(19.18, 27.98)	(1.65, 13.88)	(8.28, 23.35)	
Week 2	LS Mean (SEM)	31.32 (1.972)	5.87 (2.848)	25.45 (3.467)	<0.0001
	95% CI for LS MEAN	(27.34, 35.30)	(0.12, 11.61)	(18.46, 32.44)	
Week 3	LS Mean (SEM)	31.04 (2.062)	4.08 (3.161)	26.96 (3.779)	<0.0001
	95% CI for LS MEAN	(26.87, 35.20)	(-2.30, 10.46)	(19.33, 34.58)	
Week 4	LS Mean (SEM)	30.39 (2.908)	2.46 (4.823)	27.93 (5.632)	<0.0001
	95% CI for LS MEAN	(24.51, 36.28)	(-7.27, 12.19)	(16.56, 39.30)	
Week 6	LS Mean (SEM)	29.96 (2.781)	-0.72 (4.887)	30.68 (5.624)	<0.0001
	95% CI for LS MEAN	(24.31, 35.62)	(-10.59, 9.15)	(19.31, 42.06)	
Week 8	LS Mean (SEM)	28.03 (2.734)	1.68 (4.574)	26.35 (5.333)	<0.0001
	95% CI for LS MEAN	(22.49, 33.58)	(-7.56, 10.92)	(15.57, 37.14)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.5
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of
Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	28.48 (2.777)	3.10 (4.396)	25.38 (5.199)	<0.0001
	95% CI for LS MEAN	(22.84, 34.12)	(-5.82, 12.01)	(14.84, 35.93)	
Week 12	LS Mean (SEM)	27.97 (2.732)	0.45 (4.382)	27.52 (5.169)	<0.0001
	95% CI for LS MEAN	(22.45, 33.49)	(-8.39, 9.29)	(17.08, 37.95)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.5
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of
Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	19.55 (2.042)	12.73 (2.898)	6.82 (3.451)	0.0588
	95% CI for LS MEAN	(15.37, 23.73)	(6.78, 18.68)	(-0.27, 13.92)	
Week 2	LS Mean (SEM)	23.83 (1.986)	12.18 (3.109)	11.65 (3.623)	0.0030
	95% CI for LS MEAN	(19.78, 27.88)	(5.85, 18.51)	(4.27, 19.04)	
Week 3	LS Mean (SEM)	28.33 (2.511)	10.79 (4.062)	17.54 (4.718)	0.0007
	95% CI for LS MEAN	(23.21, 33.45)	(2.55, 19.03)	(7.95, 27.13)	
Week 4	LS Mean (SEM)	32.23 (2.918)	6.73 (4.800)	25.51 (5.566)	<0.0001
	95% CI for LS MEAN	(26.26, 38.20)	(-3.07, 16.52)	(14.14, 36.87)	
Week 6	LS Mean (SEM)	31.86 (2.312)	7.54 (3.220)	24.33 (3.915)	<0.0001
	95% CI for LS MEAN	(27.12, 36.61)	(0.91, 14.16)	(16.27, 32.38)	
Week 8	LS Mean (SEM)	29.56 (2.825)	10.01 (4.026)	19.54 (4.883)	0.0004
	95% CI for LS MEAN	(23.78, 35.33)	(1.77, 18.26)	(9.54, 29.55)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.5
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of
Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	29.71 (2.623)	7.28 (3.960)	22.43 (4.637)	<0.0001
	95% CI for LS MEAN	(24.32, 35.11)	(-0.84, 15.40)	(12.89, 31.98)	
Week 12	LS Mean (SEM)	29.59 (2.449)	10.37 (3.784)	19.22 (4.397)	0.0003
	95% CI for LS MEAN	(24.50, 34.67)	(2.52, 18.23)	(10.07, 28.37)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.5
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of
Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.1488
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.6
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Background C5 Inhibitor Therapy
(Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 11)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	20.81 (2.410)	6.74 (3.386)	14.07 (4.172)	0.0023
	95% CI for LS MEAN	(15.86, 25.76)	(-0.21, 13.69)	(5.51, 22.64)	
Week 2	LS Mean (SEM)	26.94 (2.968)	7.16 (4.071)	19.78 (5.034)	0.0006
	95% CI for LS MEAN	(20.83, 33.04)	(-1.22, 15.53)	(9.43, 30.14)	
Week 3	LS Mean (SEM)	26.21 (3.181)	6.55 (4.398)	19.66 (5.416)	0.0012
	95% CI for LS MEAN	(19.67, 32.75)	(-2.48, 15.57)	(8.54, 30.78)	
Week 4	LS Mean (SEM)	28.28 (3.291)	4.58 (4.838)	23.70 (5.831)	0.0004
	95% CI for LS MEAN	(21.50, 35.06)	(-5.36, 14.52)	(11.72, 35.68)	
Week 6	LS Mean (SEM)	29.22 (3.249)	4.63 (4.574)	24.58 (5.565)	0.0002
	95% CI for LS MEAN	(22.45, 35.98)	(-4.86, 14.12)	(13.02, 36.15)	
Week 8	LS Mean (SEM)	26.57 (3.458)	2.50 (5.272)	24.07 (6.233)	0.0011
	95% CI for LS MEAN	(19.26, 33.88)	(-8.53, 13.53)	(10.98, 37.16)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.6
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Background C5 Inhibitor Therapy
(Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 11)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	23.83 (3.164)	3.50 (4.435)	20.33 (5.373)	0.0021
	95% CI for LS MEAN	(17.02, 30.63)	(-6.03, 13.03)	(8.78, 31.88)	
Week 12	LS Mean (SEM)	26.12 (3.965)	0.67 (5.493)	25.45 (6.733)	0.0020
	95% CI for LS MEAN	(17.68, 34.55)	(-11.12, 12.46)	(11.04, 39.86)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.6
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Background C5 Inhibitor Therapy
(Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	21.19 (1.870)	10.46 (2.812)	10.73 (3.371)	0.0030
	95% CI for LS MEAN	(17.40, 24.97)	(4.75, 16.17)	(3.89, 17.57)	
Week 2	LS Mean (SEM)	27.69 (1.918)	7.64 (3.153)	20.04 (3.687)	<0.0001
	95% CI for LS MEAN	(23.81, 31.56)	(1.29, 13.99)	(12.61, 27.47)	
Week 3	LS Mean (SEM)	30.98 (2.091)	6.21 (3.565)	24.78 (4.130)	<0.0001
	95% CI for LS MEAN	(26.76, 35.21)	(-0.97, 13.38)	(16.46, 33.10)	
Week 4	LS Mean (SEM)	32.60 (2.530)	5.55 (4.467)	27.06 (5.129)	<0.0001
	95% CI for LS MEAN	(27.47, 37.74)	(-3.47, 14.56)	(16.70, 37.42)	
Week 6	LS Mean (SEM)	31.77 (2.574)	2.69 (4.470)	29.08 (5.153)	<0.0001
	95% CI for LS MEAN	(26.55, 36.99)	(-6.32, 11.70)	(18.68, 39.49)	
Week 8	LS Mean (SEM)	28.68 (2.788)	6.24 (4.633)	22.45 (5.403)	0.0001
	95% CI for LS MEAN	(23.06, 34.30)	(-3.09, 15.56)	(11.56, 33.33)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.6
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Background C5 Inhibitor Therapy
(Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	30.37 (2.568)	4.26 (4.247)	26.11 (4.957)	<0.0001
	95% CI for LS MEAN	(25.18, 35.56)	(-4.31, 12.82)	(16.12, 36.11)	
Week 12	LS Mean (SEM)	29.47 (2.557)	5.99 (4.300)	23.48 (4.995)	<0.0001
	95% CI for LS MEAN	(24.31, 34.63)	(-2.66, 14.64)	(13.42, 33.54)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Data cutoff date of 31Mar2023

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Table GERMANY.2.1.1.1.6
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Background C5 Inhibitor Therapy
(Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.9606
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Table GERMANY.2.1.1.1.7
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	21.19 (8.246)	5.90 (10.638)	15.29 (16.228)	0.3798
	95% CI for LS MEAN	(2.29, 40.08)	(-19.45, 31.25)	(-23.70, 54.28)	
Week 2	LS Mean (SEM)	26.53 (8.126)	6.10 (10.713)	20.43 (16.154)	0.2500
	95% CI for LS MEAN	(7.69, 45.37)	(-19.15, 31.35)	(-18.49, 59.36)	
Week 3	LS Mean (SEM)	27.50 (8.168)	3.39 (10.697)	24.11 (16.164)	0.1832
	95% CI for LS MEAN	(8.63, 46.36)	(-21.85, 28.62)	(-14.81, 63.03)	
Week 4	LS Mean (SEM)	30.91 (8.156)	-2.88 (10.674)	33.79 (16.142)	0.0788
	95% CI for LS MEAN	(12.04, 49.78)	(-28.18, 22.41)	(-5.20, 72.79)	
Week 6	LS Mean (SEM)	29.14 (8.048)	-2.51 (12.886)	31.65 (17.874)	0.1096
	95% CI for LS MEAN	(10.33, 47.95)	(-30.21, 25.19)	(-8.63, 71.94)	
Week 8	LS Mean (SEM)	34.24 (8.164)	0.79 (11.604)	33.45 (17.193)	0.0900
	95% CI for LS MEAN	(15.23, 53.24)	(-25.65, 27.23)	(-6.67, 73.56)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.7
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	28.35 (8.135)	1.64 (10.795)	26.71 (16.240)	0.1472
	95% CI for LS MEAN	(9.45, 47.24)	(-23.68, 26.96)	(-12.28, 65.70)	
Week 12	LS Mean (SEM)	29.16 (7.912)	0.69 (10.791)	28.47 (16.084)	0.1251
	95% CI for LS MEAN	(10.48, 47.84)	(-24.70, 26.09)	(-10.50, 67.43)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Table GERMANY.2.1.1.1.7
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 21)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	21.81 (1.624)	8.59 (2.395)	13.22 (2.865)	<0.0001
	95% CI for LS MEAN	(18.57, 25.06)	(3.80, 13.39)	(7.49, 18.95)	
Week 2	LS Mean (SEM)	27.77 (1.763)	6.77 (2.700)	21.00 (3.192)	<0.0001
	95% CI for LS MEAN	(24.24, 31.29)	(1.37, 12.16)	(14.63, 27.38)	
Week 3	LS Mean (SEM)	30.08 (1.938)	6.17 (3.084)	23.90 (3.611)	<0.0001
	95% CI for LS MEAN	(26.20, 33.95)	(0.02, 12.33)	(16.69, 31.12)	
Week 4	LS Mean (SEM)	31.15 (2.280)	6.19 (3.846)	24.96 (4.444)	<0.0001
	95% CI for LS MEAN	(26.57, 35.72)	(-1.50, 13.89)	(16.06, 33.86)	
Week 6	LS Mean (SEM)	31.29 (1.901)	4.28 (3.006)	27.01 (3.527)	<0.0001
	95% CI for LS MEAN	(27.47, 35.10)	(-1.75, 10.31)	(19.93, 34.08)	
Week 8	LS Mean (SEM)	28.62 (2.347)	5.09 (3.799)	23.54 (4.438)	<0.0001
	95% CI for LS MEAN	(23.92, 33.32)	(-2.51, 12.69)	(14.65, 32.42)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.7
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 21)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	29.43 (2.307)	4.89 (3.700)	24.54 (4.327)	<0.0001
	95% CI for LS MEAN	(24.79, 34.06)	(-2.55, 12.33)	(15.83, 33.24)	
Week 12	LS Mean (SEM)	29.00 (2.060)	4.99 (3.231)	24.00 (3.791)	<0.0001
	95% CI for LS MEAN	(24.86, 33.13)	(-1.49, 11.48)	(16.39, 31.62)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Table GERMANY.2.1.1.1.7
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of
Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.1925
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Table GERMANY.2.1.1.1.8
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	20.76 (2.204)	7.72 (3.166)	13.04 (3.825)	0.0024
	95% CI for LS MEAN	(16.20, 25.32)	(1.17, 14.27)	(5.12, 20.96)	
Week 2	LS Mean (SEM)	27.37 (1.866)	7.42 (2.985)	19.95 (3.502)	<0.0001
	95% CI for LS MEAN	(23.52, 31.22)	(1.30, 13.53)	(12.77, 27.14)	
Week 3	LS Mean (SEM)	30.79 (2.092)	3.62 (3.281)	27.17 (3.888)	<0.0001
	95% CI for LS MEAN	(26.45, 35.12)	(-3.14, 10.38)	(19.15, 35.18)	
Week 4	LS Mean (SEM)	32.28 (2.893)	1.21 (4.673)	31.06 (5.474)	<0.0001
	95% CI for LS MEAN	(26.29, 38.26)	(-8.37, 10.80)	(19.81, 42.32)	
Week 6	LS Mean (SEM)	31.64 (3.235)	-0.97 (5.300)	32.61 (6.192)	<0.0001
	95% CI for LS MEAN	(24.97, 38.32)	(-11.79, 9.86)	(19.93, 45.30)	
Week 8	LS Mean (SEM)	34.73 (2.613)	2.65 (4.203)	32.08 (4.909)	<0.0001
	95% CI for LS MEAN	(29.32, 40.13)	(-5.99, 11.29)	(21.96, 42.19)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.8
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	33.63 (2.864)	2.05 (4.389)	31.58 (5.223)	<0.0001
	95% CI for LS MEAN	(27.69, 39.57)	(-7.02, 11.11)	(20.77, 42.39)	
Week 12	LS Mean (SEM)	33.41 (3.069)	5.37 (4.851)	28.05 (5.721)	<0.0001
	95% CI for LS MEAN	(27.10, 39.73)	(-4.55, 15.28)	(16.33, 39.76)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.8
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	21.17 (2.352)	7.64 (3.805)	13.54 (4.470)	0.0047
	95% CI for LS MEAN	(16.39, 25.95)	(-0.10, 15.37)	(4.45, 22.62)	
Week 2	LS Mean (SEM)	27.64 (2.658)	7.68 (4.433)	19.95 (5.164)	0.0005
	95% CI for LS MEAN	(22.24, 33.03)	(-1.31, 16.68)	(9.48, 30.43)	
Week 3	LS Mean (SEM)	27.84 (2.925)	6.21 (5.012)	21.63 (5.792)	0.0007
	95% CI for LS MEAN	(21.89, 33.80)	(-3.97, 16.40)	(9.85, 33.41)	
Week 4	LS Mean (SEM)	29.24 (3.440)	3.10 (6.153)	26.14 (7.033)	0.0008
	95% CI for LS MEAN	(22.21, 36.27)	(-9.44, 15.63)	(11.81, 40.48)	
Week 6	LS Mean (SEM)	27.86 (3.063)	3.25 (5.302)	24.62 (6.128)	0.0004
	95% CI for LS MEAN	(21.57, 34.15)	(-7.62, 14.11)	(12.05, 37.19)	
Week 8	LS Mean (SEM)	24.69 (3.772)	3.41 (6.269)	21.28 (7.284)	0.0065
	95% CI for LS MEAN	(16.99, 32.38)	(-9.39, 16.20)	(6.41, 36.15)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.8
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	22.04 (4.023)	7.75 (6.556)	14.30 (7.685)	0.0746
	95% CI for LS MEAN	(13.78, 30.30)	(-5.77, 21.26)	(-1.53, 30.12)	
Week 12	LS Mean (SEM)	23.88 (3.079)	-1.32 (5.171)	25.20 (6.013)	0.0002
	95% CI for LS MEAN	(17.58, 30.19)	(-11.89, 9.25)	(12.90, 37.50)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.1.1.1.8
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 5)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	23.61 (6.346)	12.07 (7.399)	11.54 (9.459)	0.2363
	95% CI for LS MEAN	(10.27, 36.95)	(-3.32, 27.45)	(-8.17, 31.25)	
Week 2	LS Mean (SEM)	25.84 (5.873)	4.42 (6.557)	21.42 (8.426)	0.0214
	95% CI for LS MEAN	(13.29, 38.39)	(-9.30, 18.15)	(3.60, 39.23)	
Week 3	LS Mean (SEM)	30.47 (5.910)	11.01 (7.561)	19.46 (9.244)	0.0466
	95% CI for LS MEAN	(17.85, 43.09)	(-4.42, 26.44)	(0.31, 38.60)	
Week 4	LS Mean (SEM)	30.49 (5.978)	14.86 (7.573)	15.63 (9.331)	0.1075
	95% CI for LS MEAN	(17.78, 43.19)	(-0.60, 30.32)	(-3.67, 34.93)	
Week 6	LS Mean (SEM)	28.93 (5.891)	10.07 (6.602)	18.86 (8.591)	0.0425
	95% CI for LS MEAN	(16.35, 41.51)	(-3.74, 23.89)	(0.72, 37.00)	
Week 8	LS Mean (SEM)	19.77 (6.371)	17.31 (10.121)	2.46 (11.715)	0.8353
	95% CI for LS MEAN	(6.39, 33.15)	(-3.15, 37.78)	(-21.38, 26.29)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

Run Date: 2024-04-26T15:49:19

Data cutoff date of 31Mar2023

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Table GERMANY.2.1.1.1.8
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 5)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	28.95 (6.127)	10.30 (9.029)	18.65 (10.592)	0.0882
	95% CI for LS MEAN	(15.98, 41.92)	(-7.97, 28.57)	(-2.96, 40.26)	
Week 12	LS Mean (SEM)	26.19 (5.898)	11.38 (7.035)	14.81 (8.733)	0.1067
	95% CI for LS MEAN	(13.61, 38.78)	(-3.19, 25.95)	(-3.50, 33.13)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:49:19

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Table GERMANY.2.1.1.1.8
Change from Baseline in Hemoglobin (g/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.7531
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

The difference of LSM is calculated as the Danicopan mean minus Placebo mean.

Mixed Model for Repeated Measures (MMRM) includes the fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline Hgb value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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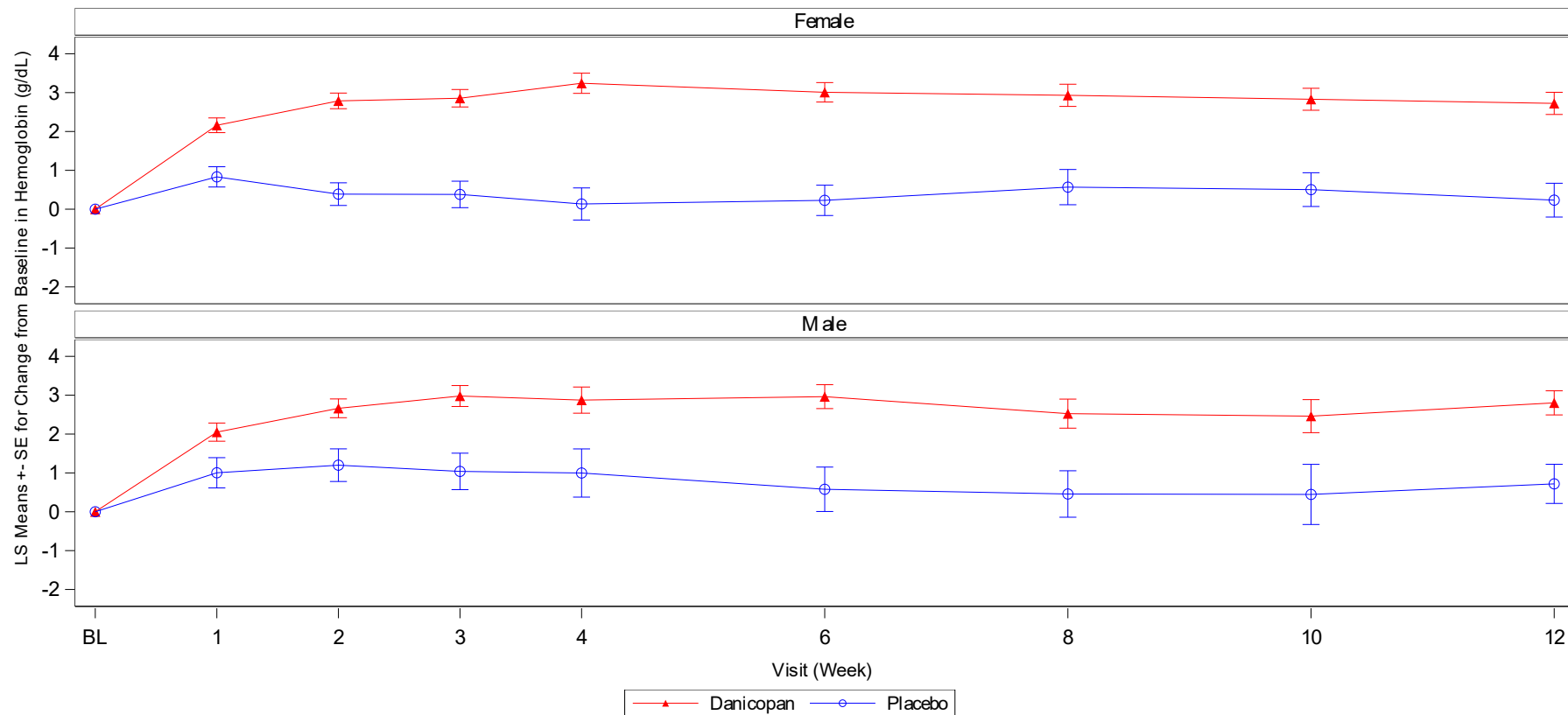
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Figure GERMANY.2.1.1.1.2

Plots of LS Means \pm SE for Change from Baseline in Hemoglobin (g/dL) During 12-Week of Treatment Period 1 by Sex
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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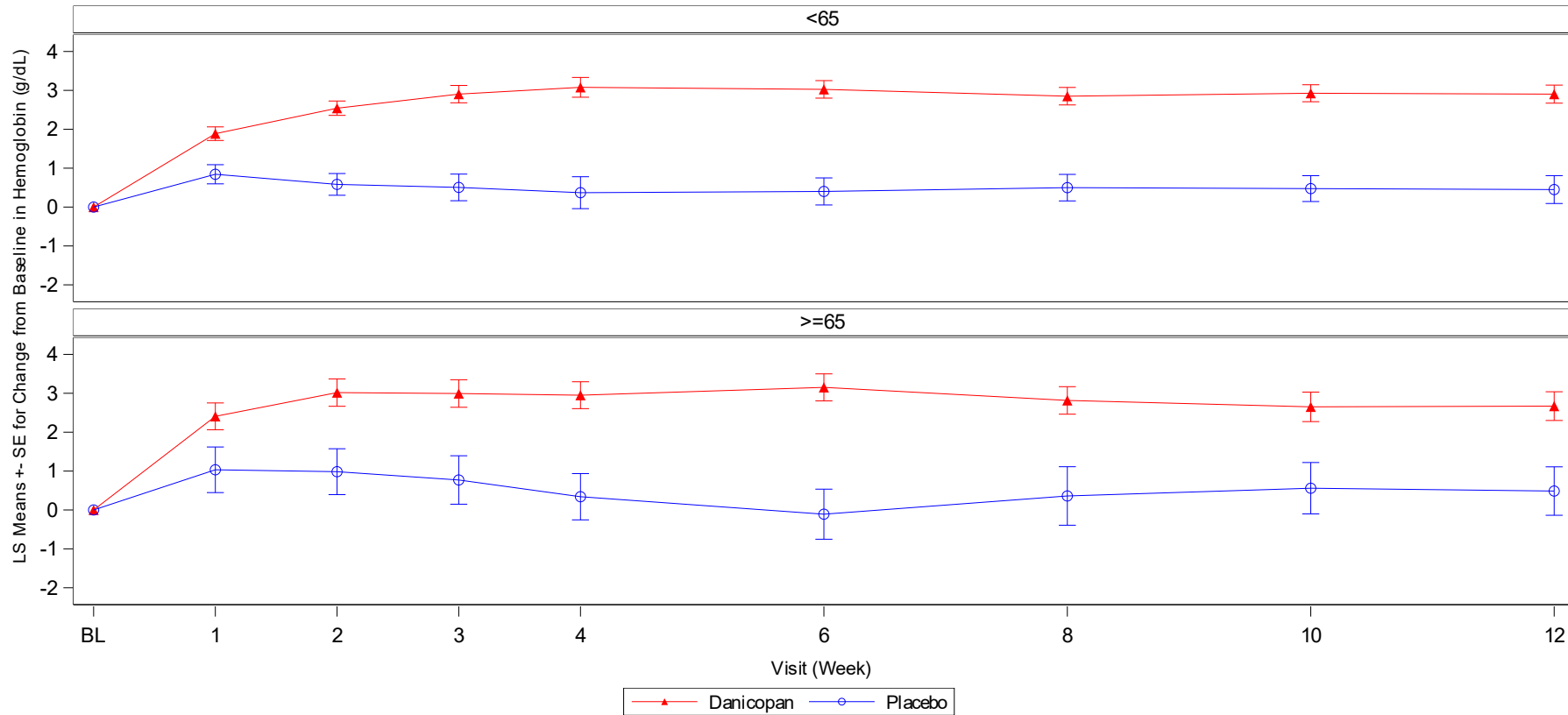
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Figure GERMANY.2.1.1.1.3
Plots of LS Means \pm SE for Change from Baseline in Hemoglobin (g/dL) During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and \geq 65 Years)
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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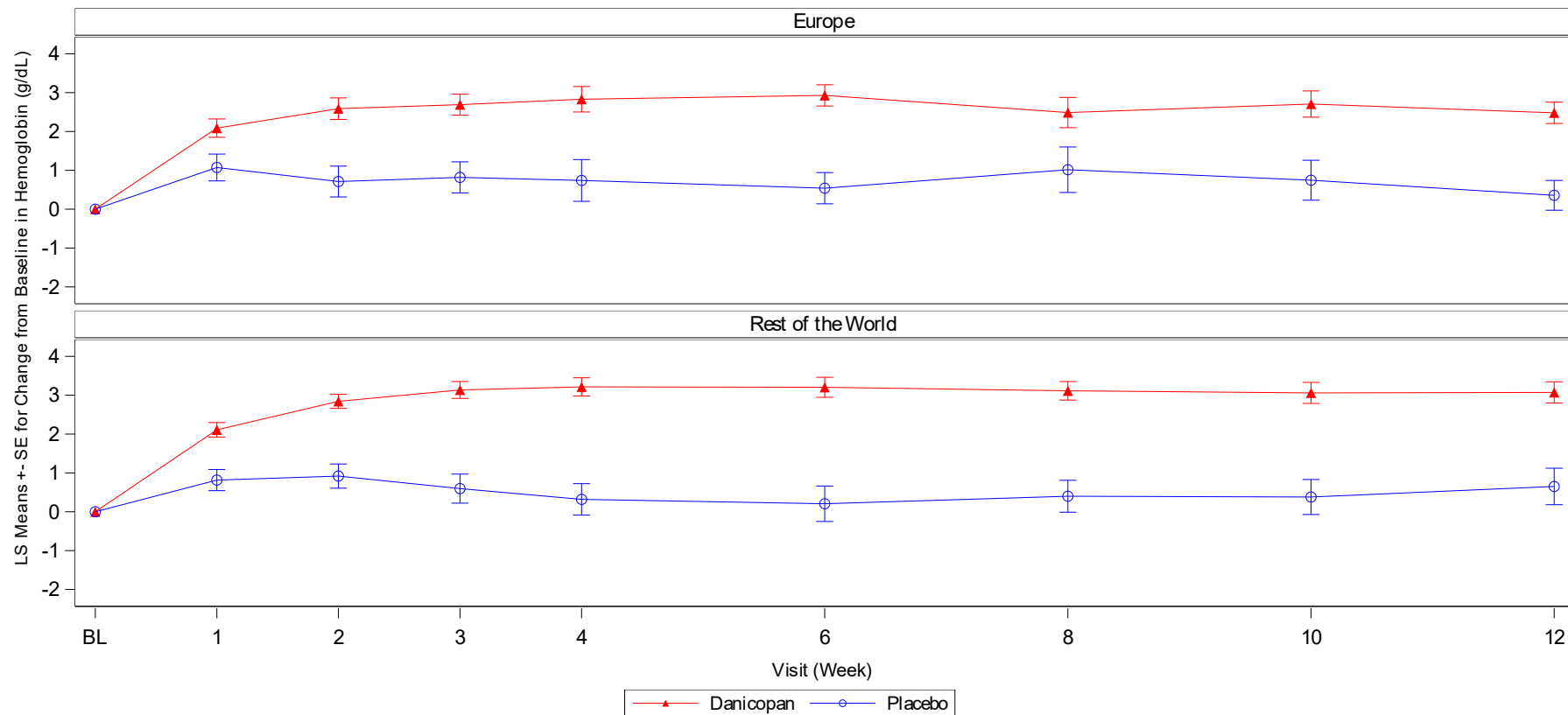
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Figure GERMANY.2.1.1.1.4

Plots of LS Means \pm SE for Change from Baseline in Hemoglobin (g/dL) During 12-Week of Treatment Period 1 by Region
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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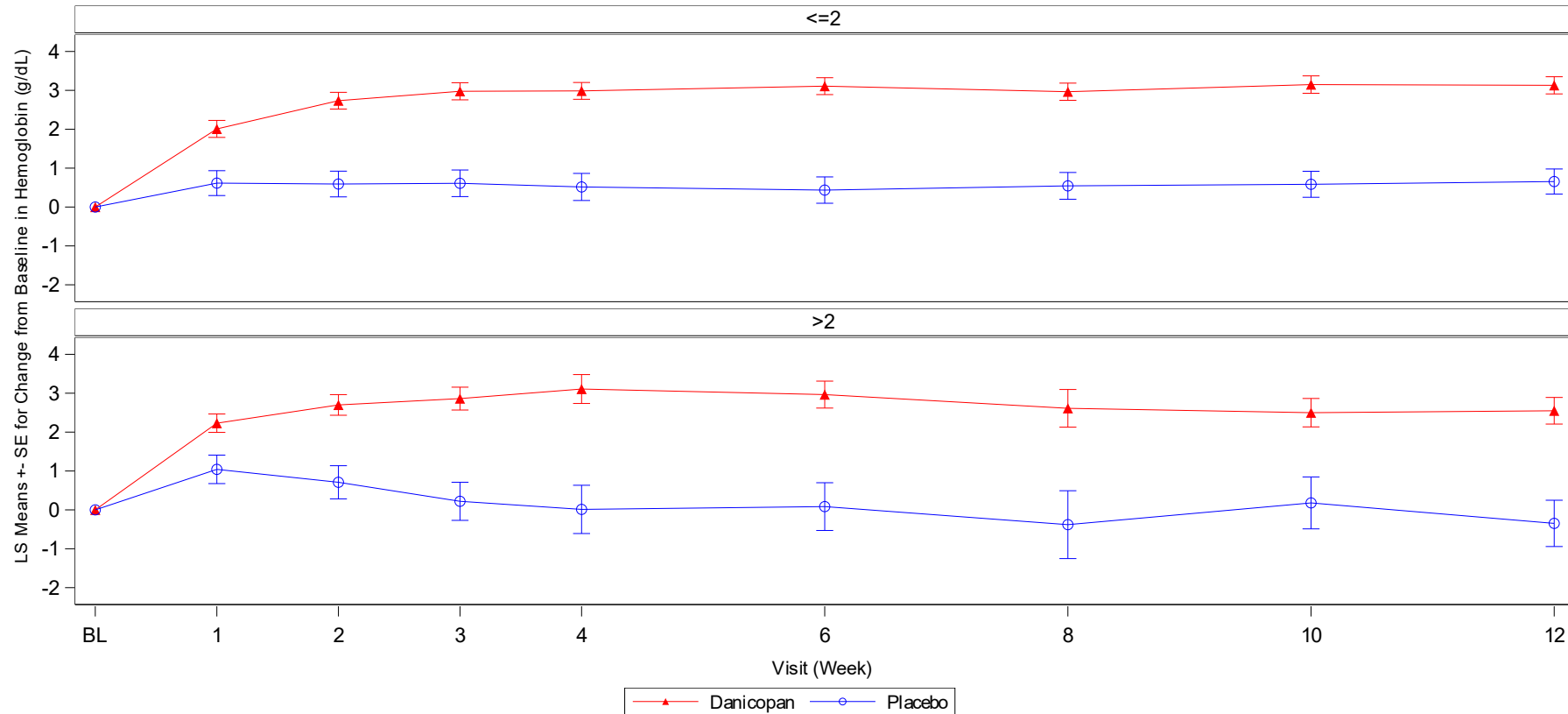
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Figure GERMANY.2.1.1.1.5

Plots of LS Means \pm SE for Change from Baseline in Hemoglobin (g/dL) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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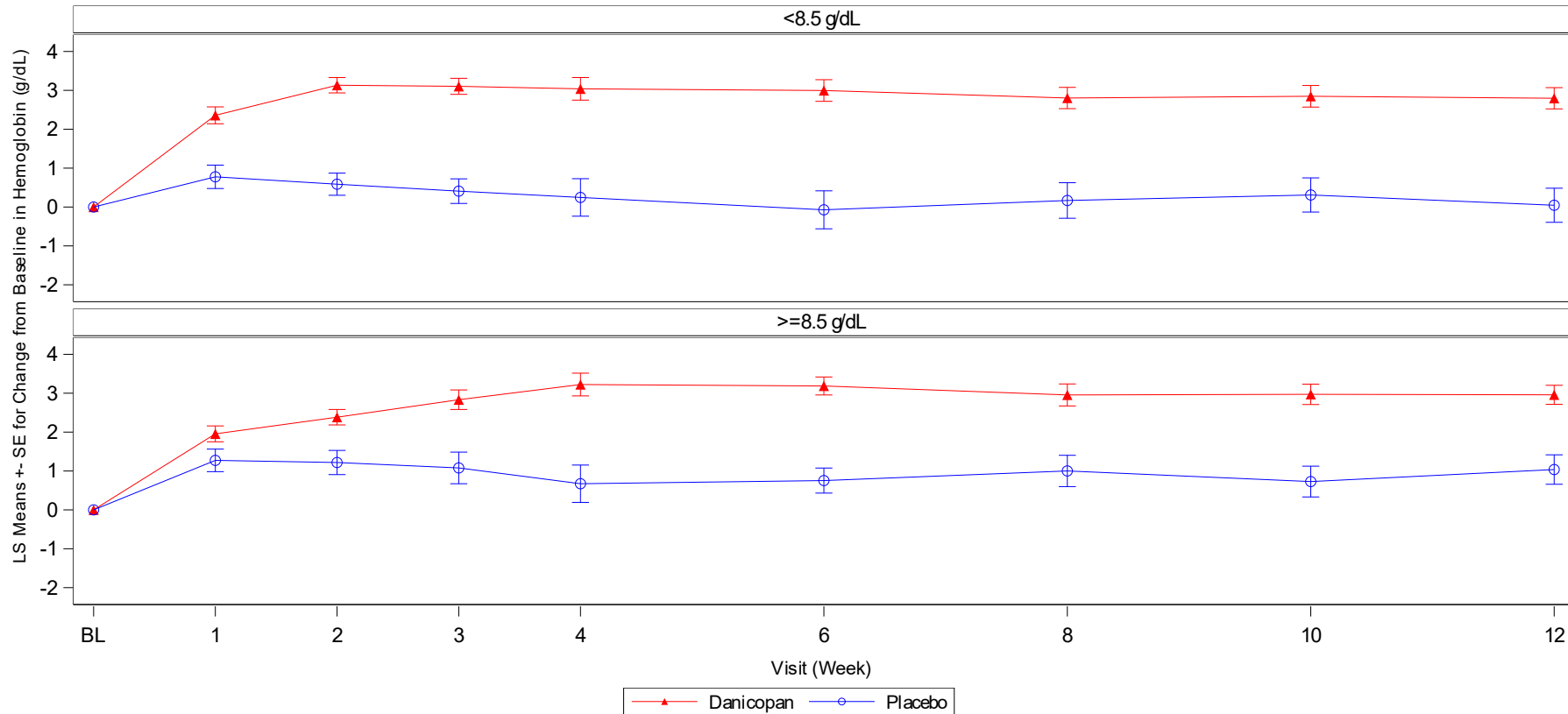
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Figure GERMANY.2.1.1.1.6
Plots of LS Means \pm SE for Change from Baseline in Hemoglobin (g/dL) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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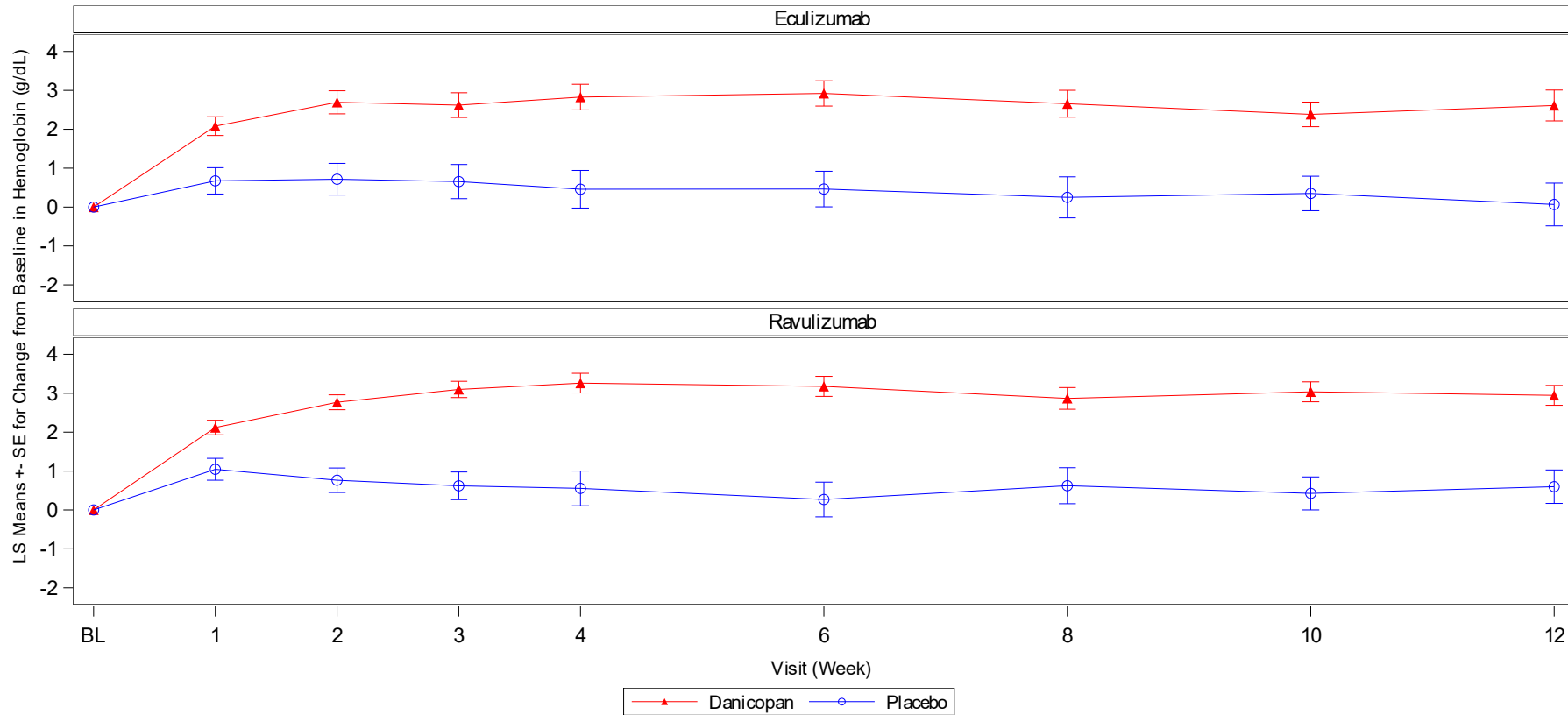
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Figure GERMANY.2.1.1.1.7
Plots of LS Means \pm SE for Change from Baseline in Hemoglobin (g/dL) During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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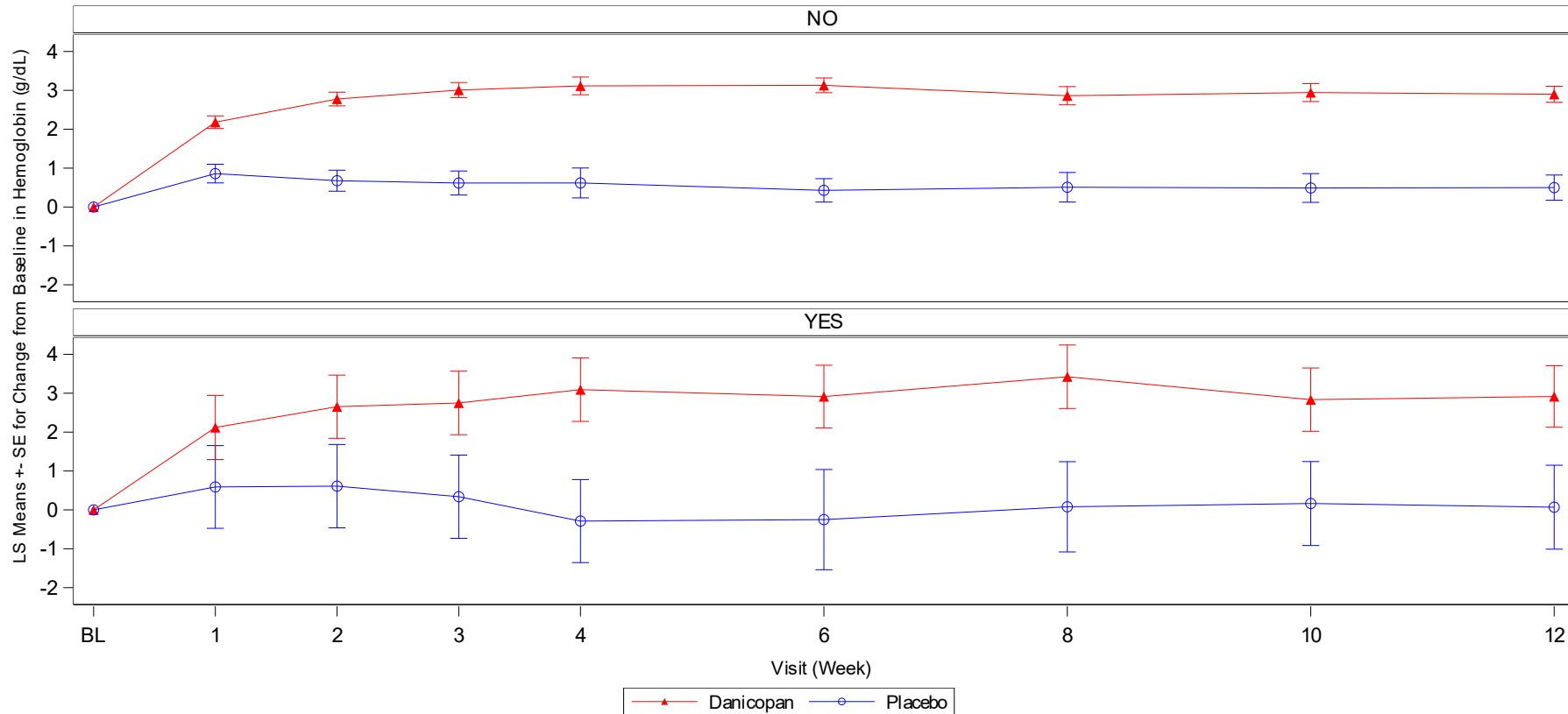
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Figure GERMANY.2.1.1.1.8
Plots of LS Means \pm SE for Change from Baseline in Hemoglobin (g/dL) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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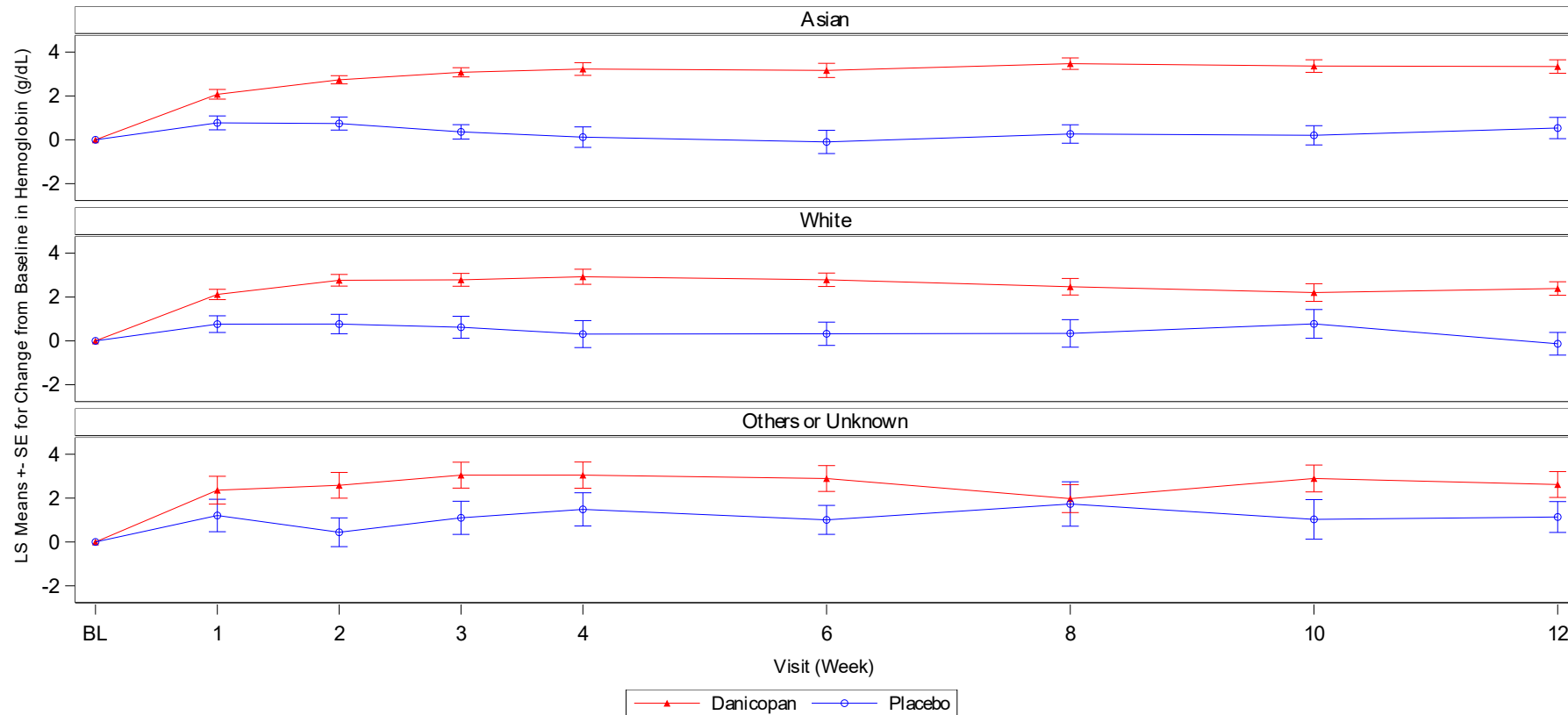
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Figure GERMANY.2.1.1.1.9

Plots of LS Means \pm SE for Change from Baseline in Hemoglobin (g/dL) During 12-Week of Treatment Period 1 by Race
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the lowest Hgb value observed between and including Screening and Day 1.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

Hgb values collected within 4 weeks after transfusion are not included in the MMRM model.

Source: ADaM.ADSL, ADaM.ADEFF

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Data cutoff date of 31Mar2023

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.1 Hämoglobinwert-assoziierte Endpunkte

1.1.2 Anstieg des Hämoglobinwertes von ≥ 2 g/dl bei gleichzeitigem Fehlen von Transfusionen

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Table GERMANY.2.2.1.1
Proportion of Patients with Hgb Increase of ≥ 2 g/dL at Week 12 in the Absence of Transfusion
During 12-Week of Treatment Period 1
Modified Randomized Set

Variable/Statistics	Danicopan (N = 57)	Placebo (N = 26)	Treatment Difference (Danicopan-Placebo)
Patients with Hgb increase of ≥ 2 g/dL at Week 12 in the absence of transfusion			
Number of Patients (n)	31	0	
Percentage (%)	54.4	0.0	46.9
95% CI	(40.66, 67.64)	(0.00, 13.23)	(31.45, 62.36)
Stratified CMH P-value			<.0001
Odds Ratio (95% CI)			NA
Relative Risk (95% CI)			NA

CI=Confidence Interval

The criterion is defined as ≥ 2 g/dL increase in Hgb from baseline to Week 12 and remaining transfusion free during 12-Week Treatment Period 1. Patients who withdraw from the study early during the 12-Week Treatment Period 1 or have missing Hgb value at Week 12 are considered as not achieving the criterion.

Percentages are based on the total number of patients in each group.

95% CI for difference between treatment is produced using the Miettinen and Nurminen method. 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for odds ratio and relative risk.

P-value are from Cochran-Mantel-Haenszel test controlling for stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Table GERMANY.2.4.9.1
Proportion of Patients with Hgb Increase of ≥ 2 g/dL at Week 24 in the Absence of Transfusion
During 24 Weeks of Treatment
Modified Randomized Set

Variable/Statistics	D -> D (N = 55)
Patients with Hgb increase of ≥ 2 g/dL at Week 24 in the absence of transfusion	
Number of Patients (n)	23
Percentage (%)	41.8
95% CI	(28.65, 55.89)

CI=Confidence Interval; N=number of patients who entered Treatment Period 2.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2

The criterion is defined as ≥ 2 g/dL increase in Hgb from baseline to week 24 and remaining transfusion free during 24-week Treatment.

Patients who withdraw from the study early during Treatment Period 2 or have missing Hgb value at Week 24 are considered as not achieving the criterion.

CI are 95% exact confidence interval, computed using Clopper-Pearson method.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:49:12

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Table GERMANY.2.4.14.1
Proportion of Patients with Hgb Increase of ≥ 2 g/dL at Week 48
Modified Randomized Set

Variable/Statistics	D -> D (N = 39)
Patients with Hgb increase of ≥ 2 g/dL at Week 48 in the absence of transfusion	
Number of Patients (n)	20
Percentage (%)	51.3
95% CI	(34.78, 67.58)

CI=Confidence Interval; N=number of patients who entered Long Term Extension Period and achieve Week 48.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2

The criterion is defined as ≥ 2 g/dL increase in Hgb from baseline to week 48, and remaining transfusion free within 4 weeks before Week 48 Hgb collected. Patients who withdraw from the study early during 48 weeks or have missing Hgb value at Week 48 are considered as not achieving the criterion.

CI are 95% exact confidence interval, computed using Clopper-Pearson method.

% = $100 \cdot n/N$

Source: ADaM.ADSL, ADaM.ADEFF

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Data cutoff date of 31Mar2023

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Table GERMANY.2.4.15.1
Proportion of Patients with Hgb Increase of ≥ 2 g/dL at Week 72
Modified Randomized Set

Variable/Statistics	D -> D (N = 20)
Patients with Hgb increase of ≥ 2 g/dL at Week 72 in the absence of transfusion	
Number of Patients (n)	12
Percentage (%)	60.0
95% CI	(36.05, 80.88)

CI=Confidence Interval; N=number of patients who entered Long Term Extension Period and achieve Week 72.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2

The criterion is defined as ≥ 2 g/dL increase in Hgb from baseline to week 72, and remaining transfusion free within 4 weeks before Week 72 Hgb collected. Patients who withdraw from the study early during 72 weeks or have missing Hgb value at Week 72 are considered as not achieving the criterion.

CI are 95% exact confidence interval, computed using Clopper-Pearson method.

% = $100 \cdot n/N$

Source: ADaM.ADSL, ADaM.ADEFF

Run Date: 2024-04-26T15:49:03

Data cutoff date of 31Mar2023

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.1 Hämoglobinwert-assoziierte Endpunkte

1.1.2 Anstieg des Hämoglobinwertes von ≥ 2 g/dl bei gleichzeitigem Fehlen von Transfusionen

1.1.2.1 Subgruppenanalysen – Anstieg des Hämoglobinwertes von ≥ 2 g/dl bei gleichzeitigem Fehlen von Transfusionen

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Table GERMANY.2.2.1.1.1
Proportion of Patients with Hgb Increase of ≥ 2 g/dL at Week 12 in the Absence of Transfusion
During 12-Week of Treatment Period 1 by Sex
Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Patients with Hgb increase of ≥ 2 g/dL at Week 12 in the absence of transfusion							NA
Male (N=32)	m=23 16 (69.6) (47.1, 86.8)	m= 9 0 (0.0, 33.6)	NA	NA	0.70 (0.30, 0.87)	0.0008	
Female (N=51)	m=34 15 (44.1) (27.2, 62.1)	m=17 0 (0.0, 19.5)	NA	NA	0.44 (0.18, 0.62)	0.0008	

CI=Confidence Interval, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference. The criterion is defined as ≥ 2 g/dL increase in Hgb from baseline to Week 12 and remaining transfusion free during 12-Week Treatment Period 1. Patients who withdraw from the study early during the 12-Week Treatment Period 1 or have missing Hgb value at Week 12 are considered as not achieving the criterion.

Percentages are based on the total number of patients in each group.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.2.1.1.2
Proportion of Patients with Hgb Increase of ≥ 2 g/dL at Week 12 in the Absence of Transfusion
During 12-Week of Treatment Period 1 by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Patients with Hgb increase of ≥ 2 g/dL at Week 12 in the absence of transfusion							NA
< 65 Years (N=61)	m=41 23 (56.1) (39.7, 71.5)	m=20 0 (0.0, 16.8)	NA	NA	0.56 (0.36, 0.72)	<.0001	
≥ 65 Years (N=22)	m=16 8 (50.0) (24.7, 75.3)	m= 6 0 (0.0, 45.9)	NA	NA	0.50 (-0.01, 0.76)	0.0511	

CI=Confidence Interval, m = number of patients in each subgroup and treatment group, % = $n/m*100$, RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference. The criterion is defined as ≥ 2 g/dL increase in Hgb from baseline to Week 12 and remaining transfusion free during 12-Week Treatment Period 1. Patients who withdraw from the study early during the 12-Week Treatment Period 1 or have missing Hgb value at Week 12 are considered as not achieving the criterion.

Percentages are based on the total number of patients in each group.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

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Table GERMANY.2.2.1.1.3
Proportion of Patients with Hgb Increase of ≥ 2 g/dL at Week 12 in the Absence of Transfusion
During 12-Week of Treatment Period 1 by Region
Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Patients with Hgb increase of ≥ 2 g/dL at Week 12 in the absence of transfusion							NA
Europe (N=38)	m=25 10 (40.0) (21.1, 61.3)	m=13 0 (0.0, 24.7)	NA	NA	0.40 (0.08, 0.61)	0.0078	
Rest of World (N=45)	m=32 21 (65.6) (46.8, 81.4)	m=13 0 (0.0, 24.7)	NA	NA	0.66 (0.39, 0.81)	<.0001	

CI=Confidence Interval, m = number of patients in each subgroup and treatment group, % = $n/m \times 100$, RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference. The criterion is defined as ≥ 2 g/dL increase in Hgb from baseline to Week 12 and remaining transfusion free during 12-Week Treatment Period 1. Patients who withdraw from the study early during the 12-Week Treatment Period 1 or have missing Hgb value at Week 12 are considered as not achieving the criterion.

Percentages are based on the total number of patients in each group.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:37:45

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Table GERMANY.2.2.1.1.4
Proportion of Patients with Hgb Increase of ≥ 2 g/dL at Week 12 in the Absence of Transfusion
During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History
(> 2 or ≤ 2 Transfusions Within 6 Months of Screening)
Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Patients with Hgb increase of ≥ 2 g/dL at Week 12 in the absence of transfusion							NA
≤ 2 Transfusions (N=49)	m=33 17 (51.5) (33.5, 69.2)	m=16 0 (0.0, 20.6)	NA	NA	0.52 (0.25, 0.69)	0.0002	
> 2 Transfusions (N=34)	m=24 14 (58.3) (36.6, 77.9)	m=10 0 (0.0, 30.8)	NA	NA	0.58 (0.19, 0.78)	0.0017	

CI=Confidence Interval, m = number of patients in each subgroup and treatment group, % = $n/m*100$, RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference. The criterion is defined as ≥ 2 g/dL increase in Hgb from baseline to Week 12 and remaining transfusion free during 12-Week Treatment Period 1. Patients who withdraw from the study early during the 12-Week Treatment Period 1 or have missing Hgb value at Week 12 are considered as not achieving the criterion.

Percentages are based on the total number of patients in each group.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:37:49

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Table GERMANY.2.2.1.1.5
Proportion of Patients with Hgb Increase of ≥ 2 g/dL at Week 12 in the Absence of Transfusion During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Patients with Hgb increase of ≥ 2 g/dL at Week 12 in the absence of transfusion							NA
< 8.5 g/dL (N=50)	m=35 16 (45.7) (28.8, 63.4)	m=15 0 (0.0, 21.8)	NA	NA	0.46 (0.16, 0.63)	0.0009	
≥ 8.5 g/dL (N=33)	m=22 15 (68.2) (45.1, 86.1)	m=11 0 (0.0, 28.5)	NA	NA	0.68 (0.31, 0.86)	0.0002	

CI=Confidence Interval, m = number of patients in each subgroup and treatment group, % = $n/m*100$, RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference. The criterion is defined as ≥ 2 g/dL increase in Hgb from baseline to Week 12 and remaining transfusion free during 12-Week Treatment Period 1. Patients who withdraw from the study early during the 12-Week Treatment Period 1 or have missing Hgb value at Week 12 are considered as not achieving the criterion.

Percentages are based on the total number of patients in each group.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:37:54

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Table GERMANY.2.2.1.1.6
Proportion of Patients with Hgb Increase of ≥ 2 g/dL at Week 12 in the Absence of Transfusion
During 12-Week of Treatment Period 1 by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Patients with Hgb increase of ≥ 2 g/dL at Week 12 in the absence of transfusion							NA
Eculizumab (N=33)	m=21 7 (33.3) (14.6, 57.0)	m=12 0 (0.0, 26.5)	NA	NA	0.33 (0.03, 0.57)	0.0319	
Ravulizumab (N=50)	m=36 24 (66.7) (49.0, 81.4)	m=14 0 (0.0, 23.2)	NA	NA	0.67 (0.42, 0.82)	<.0001	

CI=Confidence Interval, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference. The criterion is defined as ≥ 2 g/dL increase in Hgb from baseline to Week 12 and remaining transfusion free during 12-Week Treatment Period 1. Patients who withdraw from the study early during the 12-Week Treatment Period 1 or have missing Hgb value at Week 12 are considered as not achieving the criterion.

Percentages are based on the total number of patients in each group.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:37:59

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Table GERMANY.2.2.1.1.7
Proportion of Patients with Hgb Increase of ≥ 2 g/dL at Week 12 in the Absence of Transfusion
During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients
(Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Patients with Hgb increase of ≥ 2 g/dL at Week 12 in the absence of transfusion							NA
Patients Enrolled from Japan Yes (N=12)	m= 8 4 (50.0) (15.7, 84.3)	m= 4 0 (0.0, 60.2)	NA	NA	0.50 (-0.16, 0.84)	0.2081	
Patients Enrolled from Japan No (N=71)	m=49 27 (55.1) (40.2, 69.3)	m=22 0 (0.0, 15.4)	NA	NA	0.55 (0.36, 0.69)	<.0001	

CI=Confidence Interval, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference. The criterion is defined as ≥ 2 g/dL increase in Hgb from baseline to Week 12 and remaining transfusion free during 12-Week Treatment Period 1. Patients who withdraw from the study early during the 12-Week Treatment Period 1 or have missing Hgb value at Week 12 are considered as not achieving the criterion.

Percentages are based on the total number of patients in each group.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:38:04

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Table GERMANY.2.2.1.1.8
Proportion of Patients with Hgb Increase of ≥ 2 g/dL at Week 12 in the Absence of Transfusion During 12-Week of Treatment Period 1 by Race
Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Patients with Hgb increase of ≥ 2 g/dL at Week 12 in the absence of transfusion							NA
Asian (N=32)	m=22 17 (77.3) (54.6, 92.2)	m=10 0 (0.0, 30.8)	NA	NA	0.77 (0.44, 0.92)	<.0001	
White (N=39)	m=28 11 (39.3) (21.5, 59.4)	m=11 0 (0.0, 28.5)	NA	NA	0.39 (0.04, 0.59)	0.0172	
Others/Unknown (N=12)	m= 7 3 (42.9) (9.9, 81.6)	m= 5 0 (0.0, 52.2)	NA	NA	0.43 (-0.17, 0.82)	0.2045	

CI=Confidence Interval, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference. The criterion is defined as ≥ 2 g/dL increase in Hgb from baseline to Week 12 and remaining transfusion free during 12-Week Treatment Period 1. Patients who withdraw from the study early during the 12-Week Treatment Period 1 or have missing Hgb value at Week 12 are considered as not achieving the criterion.

Percentages are based on the total number of patients in each group.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:38:10

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.1 Hämoglobinwert-assozierte Endpunkte

1.1.3 Hämoglobin-Normalisierung

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Table GERMANY.2.3.5.1
Proportion of Patients with Hemoglobin Normalization at Week 12
Modified Randomized Set

Variable/Statistics	Danicopan (N = 57)	Placebo (N = 26)	Treatment Difference (Danicopan-Placebo)
Achieving Hemoglobin Normalization at Week 12			
Number of Patients (n)	15	0	
Percentage (%)	26.3	0.0	18.4
95% CI	(15.54, 39.66)	(0.00, 13.23)	(1.84, 35.02)
Stratified CMH P-value			0.0032
Odds Ratio (95% CI)			NA
Relative Risk (95% CI)			NA

Hemoglobin normalization is defined as hemoglobin value above lower limit of normal reference range.

Percentages are based on the total number of patients in each group.

95% CI for difference between treatment is produced using the Miettinen and Nurminen method. 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for odds ratio and relative risk.

Patients with transfusions within 4 weeks prior to Week 12 are considered as not meeting hemoglobin normalization regardless of actual value observed at Week 12.

P-value are from Cochran-Mantel-Haenszel test controlling for stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:49:30

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Table GERMANY.2.4.10.1
Proportion of Patients with Hemoglobin Normalization at Week 24
Modified Randomized Set

Variable/Statistics	D -> D (N = 55)
Achieving Hemoglobin Normalization at Week 24	
Number of Patients (n)	11
Percentage (%)	20.0
95% CI	(10.43, 32.97)

CI=Confidence Interval; N=number of patients who entered Treatment Period 2.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2

Hemoglobin normalization is defined as hemoglobin value above lower limit of normal reference range.

Patients with transfusions within 4 weeks prior to Week 24 are considered as not meeting hemoglobin normalization regardless of actual value observed at Week 24.

CI are 95% exact confidence interval, computed using Clopper-Pearson method.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.4.23.1
Proportion of Patients with Hemoglobin Normalization at Week 48
Modified Randomized Set

Variable/Statistics	D -> D (N = 39)
Achieving Hemoglobin Normalization at Week 48	
Number of Patients (n)	8
Percentage (%)	20.5
95% CI	(9.30, 36.46)

CI=Confidence Interval; N=number of patients who entered Long Term Extension Period and achieve Week 48.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2
Hemoglobin normalization is defined as hemoglobin value above lower limit of normal reference range.
Patients with transfusions within 4 weeks prior to Week 48 are considered as not meeting hemoglobin normalization regardless of actual value observed at Week 48.
CI are 95% exact confidence interval, computed using Clopper-Pearson method.
% = 100*n/N
Source: ADaM.ADSL, ADaM.ADEFF
Run Date: 2024-04-26T15:49:20
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Table GERMANY.2.4.24.1
Proportion of Patients with Hemoglobin Normalization at Week 72
Modified Randomized Set

Variable/Statistics	D -> D (N = 20)
Achieving Hemoglobin Normalization at Week 72	
Number of Patients (n)	3
Percentage (%)	15.0
95% CI	(3.21, 37.89)

CI=Confidence Interval; N=number of patients who entered Long Term Extension Period and achieve Week 72.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2
Hemoglobin normalization is defined as hemoglobin value above lower limit of normal reference range.
Patients with transfusions within 4 weeks prior to Week 72 are considered as not meeting hemoglobin normalization regardless of actual value observed at Week 72.
CI are 95% exact confidence interval, computed using Clopper-Pearson method.
% = 100*n/N
Source: ADaM.ADSL, ADaM.ADEFF
Run Date: 2024-04-26T15:49:20
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Data cutoff date of 31Mar2023
FINAL

Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.1 Hämoglobinwert-assoziierte Endpunkte

1.1.3 Hämoglobin-Normalisierung

1.1.3.1 Subgruppenanalysen – Hämoglobin-Normalisierung

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Table GERMANY.2.3.5.2
Proportion of Patients with Hemoglobin Normalization at Week 12 by Sex
Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Achieving Hemoglobin Normalization at Week 12							NA
Male (N=32)	m=23 1 (4.3) (0.1, 21.9)	m= 9 0 (0.0, 33.6)	NA	NA	0.04 (-0.29, 0.23)	1.0000	
Female (N=51)	m=34 14 (41.2) (24.6, 59.3)	m=17 0 (0.0, 19.5)	NA	NA	0.41 (0.14, 0.59)	0.0018	

CI=Confidence Interval, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.
Hemoglobin normalization is defined as hemoglobin value above lower limit of normal reference range.

Percentages are based on the total number of patients in each group.

Patients with transfusions within 4 weeks prior to Week 12 are considered as not meeting hemoglobin normalization regardless of actual value observed at Week 12.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:38:16

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Table GERMANY.2.3.5.3
Proportion of Patients with Hemoglobin Normalization at Week 12 by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Achieving Hemoglobin Normalization at Week 12							NA
< 65 Years (N=61)	m=41 10 (24.4) (12.4, 40.3)	m=20 0 (0.0, 16.8)	NA	NA	0.24 (0.02, 0.40)	0.0228	
≥ 65 Years (N=22)	m=16 5 (31.3) (11.0, 58.7)	m= 6 0 (0.0, 45.9)	NA	NA	0.31 (-0.17, 0.59)	0.2663	

CI=Confidence Interval, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.
Hemoglobin normalization is defined as hemoglobin value above lower limit of normal reference range.

Percentages are based on the total number of patients in each group.

Patients with transfusions within 4 weeks prior to Week 12 are considered as not meeting hemoglobin normalization regardless of actual value observed at Week 12.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:38:22

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Table GERMANY.2.3.5.4
Proportion of Patients with Hemoglobin Normalization at Week 12 by Region
Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Achieving Hemoglobin Normalization at Week 12							NA
Europe (N=38)	m=25 4 (16.0) (4.5, 36.1)	m=13 0 (0.0, 24.7)	NA	NA	0.16 (-0.10, 0.36)	0.2779	
Rest of World (N=45)	m=32 11 (34.4) (18.6, 53.2)	m=13 0 (0.0, 24.7)	NA	NA	0.34 (0.03, 0.54)	0.0195	

CI=Confidence Interval, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.
Hemoglobin normalization is defined as hemoglobin value above lower limit of normal reference range.

Percentages are based on the total number of patients in each group.

Patients with transfusions within 4 weeks prior to Week 12 are considered as not meeting hemoglobin normalization regardless of actual value observed at Week 12.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:38:29

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Table GERMANY.2.3.5.5
Proportion of Patients with Hemoglobin Normalization at Week 12 by the Randomization Stratification Factors of
Transfusion History (> 2 or ≤ 2 Transfusions Within 6 Months of Screening)
Modified Randomized Set

Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
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This subgroup did not meet the criteria for analysis.

CI=Confidence Interval, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.
Hemoglobin normalization is defined as hemoglobin value above lower limit of normal reference range.

Percentages are based on the total number of patients in each group.

Patients with transfusions within 4 weeks prior to Week 12 are considered as not meeting hemoglobin normalization regardless of actual value observed at Week 12.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:38:35

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Table GERMANY.2.3.5.6
Proportion of Patients with Hemoglobin Normalization at Week 12 by the Randomization Stratification Factors of
Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
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This subgroup did not meet the criteria for analysis.

CI=Confidence Interval, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.
Hemoglobin normalization is defined as hemoglobin value above lower limit of normal reference range.

Percentages are based on the total number of patients in each group.

Patients with transfusions within 4 weeks prior to Week 12 are considered as not meeting hemoglobin normalization regardless of actual value observed at Week 12.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:38:35

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Table GERMANY.2.3.5.7
Proportion of Patients with Hemoglobin Normalization at Week 12 by Background C5 Inhibitor Therapy
(Eculizumab or Ravulizumab)
Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Achieving Hemoglobin Normalization at Week 12							NA
Eculizumab (N=33)	m=21 4 (19.0) (5.4, 41.9)	m=12 0 (0.0, 26.5)	NA	NA	0.19 (-0.09, 0.42)	0.2713	
Ravulizumab (N=50)	m=36 11 (30.6) (16.3, 48.1)	m=14 0 (0.0, 23.2)	NA	NA	0.31 (0.03, 0.48)	0.0221	

CI=Confidence Interval, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.
Hemoglobin normalization is defined as hemoglobin value above lower limit of normal reference range.

Percentages are based on the total number of patients in each group.

Patients with transfusions within 4 weeks prior to Week 12 are considered as not meeting hemoglobin normalization regardless of actual value observed at Week 12.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:38:44

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Table GERMANY.2.3.5.8
Proportion of Patients with Hemoglobin Normalization at Week 12 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Achieving Hemoglobin Normalization at Week 12							NA
Patients Enrolled from Japan Yes (N=12)	m= 8 2 (25.0) (3.2, 65.1)	m= 4 0 (0.0, 60.2)	NA	NA	0.25 (-0.37, 0.65)	0.5152	
Patients Enrolled from Japan No (N=71)	m=49 13 (26.5) (14.9, 41.1)	m=22 0 (0.0, 15.4)	NA	NA	0.27 (0.05, 0.41)	0.0066	

CI=Confidence Interval, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference. Hemoglobin normalization is defined as hemoglobin value above lower limit of normal reference range.

Percentages are based on the total number of patients in each group.

Patients with transfusions within 4 weeks prior to Week 12 are considered as not meeting hemoglobin normalization regardless of actual value observed at Week 12.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:38:35

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Table GERMANY.2.3.5.9
Proportion of Patients with Hemoglobin Normalization at Week 12 by Race
Modified Randomized Set

Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
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This subgroup did not meet the criteria for analysis.

CI=Confidence Interval, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.
Hemoglobin normalization is defined as hemoglobin value above lower limit of normal reference range.

Percentages are based on the total number of patients in each group.

Patients with transfusions within 4 weeks prior to Week 12 are considered as not meeting hemoglobin normalization regardless of actual value observed at Week 12.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom

31.03.2023 1 Morbidität

1.2 Transfusionsvermeidung und -bedarf

1.2.1 Transfusionsvermeidung

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Table GERMANY.2.2.2.1
Proportion of Patients Achieving Transfusion Avoidance During 12-Week of Treatment Period 1
Modified Randomized Set

Variable/Statistics	Danicopan (N = 57)	Placebo (N = 26)	Treatment Difference (Danicopan-Placebo)
Achieving pRBC/Whole Blood Transfusion Avoidance through Week 12			
Number of Patients (n)	45	8	
Percentage (%)	78.9	30.8	46.4
95% CI	(66.11, 88.62)	(14.33, 51.79)	(29.30, 63.53)
Stratified CMH P-value			<.0001
Odds Ratio (95% CI)			8.44 (2.65, 27.72)
Relative Risk (95% CI)			2.57 (1.50, 6.82)

CI=Confidence Interval; pRBC=packed red blood cells.

Transfusion Avoidance is defined as the proportion of patients who remain transfusion free and do not require a transfusion per protocol specified guidelines through Week 12.

Patients who withdraw from the study during randomized treatment period are considered as non-responders and are counted in the group requiring transfusions. Percentages are based on the total number of patients in each group.

95% CI for difference between treatment is produced using the Miettinen and Nurminen method. 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for odds ratio and relative risk.

P-value are from Cochran-Mantel-Haenszel test controlling for stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:54:11

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Table GERMANY.2.4.7.1
Proportion of Patients Achieving Transfusion Avoidance During 12-Week of Treatment Period 2
Modified Randomized Set

Variable/Statistics	D -> D (N = 55)	P -> D (N = 24)
Achieving pRBC/Whole Blood Transfusion Avoidance from Week 12 to Week 24		
Number of Patients (n)	44	20
Percentage (%)	80.0	83.3
95% CI	(67.03, 89.57)	(62.62, 95.26)

CI=Confidence Interval; pRBC=packed red blood cells; N=number of patients who entered Treatment Period 2.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D : Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Transfusion Avoidance is defined as the proportion of patients who remain transfusion free and do not require a transfusion per protocol specified guidelines from Week 12 to Week 24.

Patients who withdraw from the study during treatment period 2 are considered as non-responders and are counted in the group requiring transfusions.

Percentages are based on the total number of patients in each group.

CI are 95% exact confidence interval, computed using Clopper-Pearson method.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:54:12

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Table GERMANY.2.4.20.1
Proportion of Patients Achieving Transfusion Avoidance from Week 24 to Week 48
Modified Randomized Set

Variable/Statistics	D -> D (N = 39)	P -> D (N = 20)
Achieving pRBC/Whole Blood Transfusion Avoidance from Week 24 to Week 48		
Number of Patients (n)	35	16
Percentage (%)	89.7	80.0
95% CI	(75.78, 97.13)	(56.34, 94.27)

CI=Confidence Interval; pRBC=packed red blood cells; N=number of patients who entered Long Term Extension Period and achieve Week 48.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D : Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Transfusion Avoidance is defined as the proportion of patients who remain transfusion free and do not require a transfusion per protocol specified guidelines from Week 24 to Week 48.

Patients who withdraw from the study from Week 24 to Week 48 of Treatment are considered as non-responders and are counted in the group requiring transfusions.
CI are 95% exact confidence interval, computed using Clopper-Pearson method.

% = 100*n/N

Source: ADaM.ADSL, ADaM.ADEFF

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Data cutoff date of 31Mar2023

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Table GERMANY.2.4.21.1
Proportion of Patients Achieving Transfusion Avoidance from Week 48 to Week 72
Modified Randomized Set

Variable/Statistics	D -> D (N = 20)	P -> D (N = 12)
Achieving pRBC/Whole Blood Transfusion Avoidance from Week 48 to Week 72		
Number of Patients (n)	17	9
Percentage (%)	85.0	75.0
95% CI	(62.11, 96.79)	(42.81, 94.51)

CI=Confidence Interval; pRBC=packed red blood cells; N=number of patients who entered Long Term Extension Period and achieve Week 72.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D : Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Transfusion Avoidance is defined as the proportion of patients who remain transfusion free and do not require a transfusion per protocol specified guidelines from Week 48 to Week 72.

Patients who withdraw from the study from Week 48 to Week 72 of Treatment are considered as non-responders and are counted in the group requiring transfusions.
CI are 95% exact confidence interval, computed using Clopper-Pearson method.

% = 100*n/N

Source: ADaM.ADSL, ADaM.ADEFF

Run Date: 2024-04-26T15:54:03

Data cutoff date of 31Mar2023

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.2 Transfusionsvermeidung und -bedarf

1.2.1 Transfusionsvermeidung

1.2.1.1 Subgruppenanalysen – Transfusionsvermeidung

Post-hoc Analysen Studie ALPHA

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Table GERMANY.2.2.2.1.1
Proportion of Patients Achieving Transfusion Avoidance During 12-Week of Treatment Period 1 by Sex
Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Achieving pRBC/Whole Blood Transfusion Avoidance through Week 12							0.9442
Male (N=32)	m=23 20 (87.0) (66.4, 97.2)	m= 9 4 (44.4) (13.7, 78.8)	1.96 (1.06, 7.11)	8.33 (1.03, 72.51)	0.43 (0.05, 0.74)	0.0233	
Female (N=51)	m=34 25 (73.5) (55.6, 87.1)	m=17 4 (23.5) (6.8, 49.9)	3.13 (1.39, 16.62)	9.03 (2.00, 46.11)	0.50 (0.18, 0.72)	0.0010	

CI=Confidence Interval, pRBC=packed red blood cells, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

Transfusion Avoidance is defined as the proportion of patients who remain transfusion free and do not require a transfusion per protocol specified guidelines through Week 12.

Patients who withdraw from the study during randomized treatment period are considered as non-responders and are counted in the group requiring transfusions. Percentages are based on the total number of patients in each group.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:38:51

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Table GERMANY.2.2.2.1.2
Proportion of Patients Achieving Transfusion Avoidance During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Achieving pRBC/Whole Blood Transfusion Avoidance through Week 12							0.6245
< 65 Years (N=61)	m=41 33 (80.5) (65.1, 91.2)	m=20 7 (35.0) (15.4, 59.2)	2.30 (1.30, 7.22)	7.66 (1.99, 30.31)	0.45 (0.15, 0.68)	0.0012	
≥ 65 Years (N=22)	m=16 12 (75.0) (47.6, 92.7)	m= 6 1 (16.7) (0.4, 64.1)	4.50 (1.12, 134.41)	15.00 (1.03, 766.47)	0.58 (0.07, 0.86)	0.0231	

CI=Confidence Interval, pRBC=packed red blood cells, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

Transfusion Avoidance is defined as the proportion of patients who remain transfusion free and do not require a transfusion per protocol specified guidelines through Week 12.

Patients who withdraw from the study during randomized treatment period are considered as non-responders and are counted in the group requiring transfusions. Percentages are based on the total number of patients in each group.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:38:56

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Table GERMANY.2.2.2.1.3
Proportion of Patients Achieving Transfusion Avoidance During 12-Week of Treatment Period 1 by Region
Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Achieving pRBC/Whole Blood Transfusion Avoidance through Week 12							0.0783
Europe (N=38)	m=25 17 (68.0) (46.5, 85.1)	m=13 5 (38.5) (13.9, 68.4)	1.77 (0.92, 7.63)	3.40 (0.69, 17.49)	0.30 (-0.05, 0.59)	0.0982	
Rest of World (N=45)	m=32 28 (87.5) (71.0, 96.5)	m=13 3 (23.1) (5.0, 53.8)	3.79 (1.63, 23.78)	23.33 (3.57, 174.66)	0.64 (0.32, 0.86)	<.0001	

CI=Confidence Interval, pRBC=packed red blood cells, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

Transfusion Avoidance is defined as the proportion of patients who remain transfusion free and do not require a transfusion per protocol specified guidelines through Week 12.

Patients who withdraw from the study during randomized treatment period are considered as non-responders and are counted in the group requiring transfusions. Percentages are based on the total number of patients in each group.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.2.1.4
Proportion of Patients Achieving Transfusion Avoidance During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions Within 6 Months of Screening) Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Achieving pRBC/Whole Blood Transfusion Avoidance through Week 12							0.7508
≤ 2 Transfusions (N=49)	m=33 27 (81.8) (64.5, 93.0)	m=16 5 (31.3) (11.0, 58.7)	2.62 (1.33, 8.07)	9.90 (2.09, 49.56)	0.51 (0.15, 0.74)	0.0010	
> 2 Transfusions (N=34)	m=24 18 (75.0) (53.3, 90.2)	m=10 3 (30.0) (6.7, 65.2)	2.50 (1.05, 13.80)	7.00 (1.08, 52.51)	0.45 (0.04, 0.74)	0.0223	

CI=Confidence Interval, pRBC=packed red blood cells, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

Transfusion Avoidance is defined as the proportion of patients who remain transfusion free and do not require a transfusion per protocol specified guidelines through Week 12.

Patients who withdraw from the study during randomized treatment period are considered as non-responders and are counted in the group requiring transfusions. Percentages are based on the total number of patients in each group.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.2.1.5
Proportion of Patients Achieving Transfusion Avoidance During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Achieving pRBC/Whole Blood Transfusion Avoidance through Week 12							0.0547
< 8.5 g/dL (N=50)	m=35 24 (68.6) (50.7, 83.1)	m=15 5 (33.3) (11.8, 61.6)	2.06 (1.03, 6.38)	4.36 (1.02, 19.90)	0.35 (0.02, 0.62)	0.0299	
≥ 8.5 g/dL (N=33)	m=22 21 (95.5) (77.2, 99.9)	m=11 3 (27.3) (6.0, 61.0)	3.50 (1.60, 23.10)	56.00 (4.13, 2594.91)	0.68 (0.31, 0.90)	<.0001	

CI=Confidence Interval, pRBC=packed red blood cells, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

Transfusion Avoidance is defined as the proportion of patients who remain transfusion free and do not require a transfusion per protocol specified guidelines through Week 12.

Patients who withdraw from the study during randomized treatment period are considered as non-responders and are counted in the group requiring transfusions. Percentages are based on the total number of patients in each group.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.2.1.6
Proportion of Patients Achieving Transfusion Avoidance During 12-Week of Treatment Period 1 by
Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Achieving pRBC/Whole Blood Transfusion Avoidance through Week 12							0.2077
Eculizumab (N=33)	m=21 14 (66.7) (43.0, 85.4)	m=12 4 (33.3) (9.9, 65.1)	2.00 (0.92, 8.40)	4.00 (0.72, 24.11)	0.33 (-0.04, 0.64)	0.0827	
Ravulizumab (N=50)	m=36 31 (86.1) (70.5, 95.3)	m=14 4 (28.6) (8.4, 58.1)	3.01 (1.41, 12.93)	15.50 (2.85, 91.13)	0.58 (0.23, 0.80)	0.0002	

CI=Confidence Interval, pRBC=packed red blood cells, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

Transfusion Avoidance is defined as the proportion of patients who remain transfusion free and do not require a transfusion per protocol specified guidelines through Week 12.

Patients who withdraw from the study during randomized treatment period are considered as non-responders and are counted in the group requiring transfusions. Percentages are based on the total number of patients in each group.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.2.2.1.7
Proportion of Patients Achieving Transfusion Avoidance During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Achieving pRBC/Whole Blood Transfusion Avoidance through Week 12							0.9613
Patients Enrolled from Japan Yes (N=12)	m= 8 6 (75.0) (34.9, 96.8)	m= 4 1 (25.0) (0.6, 80.6)	3.00 (0.77, 85.77)	9.00 (0.35, 548.99)	0.50 (-0.14, 0.88)	0.2222	
Patients Enrolled from Japan No (N=71)	m=49 39 (79.6) (65.7, 89.8)	m=22 7 (31.8) (13.9, 54.9)	2.50 (1.39, 6.70)	8.36 (2.36, 30.59)	0.48 (0.21, 0.68)	0.0003	

CI=Confidence Interval, pRBC=packed red blood cells, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

Transfusion Avoidance is defined as the proportion of patients who remain transfusion free and do not require a transfusion per protocol specified guidelines through Week 12.

Patients who withdraw from the study during randomized treatment period are considered as non-responders and are counted in the group requiring transfusions. Percentages are based on the total number of patients in each group.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.2.1.8
Proportion of Patients Achieving Transfusion Avoidance During 12-Week of Treatment Period 1 by Race
Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Achieving pRBC/Whole Blood Transfusion Avoidance through Week 12							0.2527
Asian (N=32)	m=22 20 (90.9) (70.8, 98.9)	m=10 3 (30.0) (6.7, 65.2)	3.03 (1.36, 16.32)	23.33 (2.44, 289.62)	0.61 (0.19, 0.86)	0.0010	
White (N=39)	m=28 19 (67.9) (47.6, 84.1)	m=11 4 (36.4) (10.9, 69.2)	1.87 (0.92, 6.74)	3.69 (0.69, 21.31)	0.31 (-0.07, 0.62)	0.1461	

CI=Confidence Interval, pRBC=packed red blood cells, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

Transfusion Avoidance is defined as the proportion of patients who remain transfusion free and do not require a transfusion per protocol specified guidelines through Week 12.

Patients who withdraw from the study during randomized treatment period are considered as non-responders and are counted in the group requiring transfusions. Percentages are based on the total number of patients in each group.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.2.1.8
Proportion of Patients Achieving Transfusion Avoidance During 12-Week of Treatment Period 1 by Race
Modified Randomized Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Others or Unknown (N=12)	m= 7 6 (85.7) (42.1, 99.6)	m= 5 1 (20.0) (0.5, 71.6)	4.29 (1.01, 128.75)	24.00 (0.75, 1370.98)	0.66 (0.01, 0.95)	0.0720	

CI=Confidence Interval, pRBC=packed red blood cells, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

Transfusion Avoidance is defined as the proportion of patients who remain transfusion free and do not require a transfusion per protocol specified guidelines through Week 12.

Patients who withdraw from the study during randomized treatment period are considered as non-responders and are counted in the group requiring transfusions. Percentages are based on the total number of patients in each group.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients in each level and at least 10 patients with response in at least one level is summarized.

Source: ADaM.ADSL, ADaM.ADEFF

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.2 Transfusionsvermeidung und -bedarf

1.2.2 Transfundierte Erythrozyten-Einheiten und Transfusionsfälle

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Table GERMANY.2.6.5.1
Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances
from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment
Modified Randomized Set

Treatment Group : Danicopan

Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks
Number of red blood cell (RBC) units transfused	n	57	57	57
	Mean (SD)	2.035 (2.3143)	0.684 (1.5942)	-1.351 (2.3716)
	Median	1.0	0.0	-1.0
	Min, Max	0, 11	0, 7	-11, 4
Number of transfusion instances	n	57	57	57
	Mean (SD)	1.333 (1.3408)	0.456 (1.1506)	-0.877 (1.3103)
	Median	1.0	0.0	-1.0
	Min, Max	0, 6	0, 6	-4, 3

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Table GERMANY.2.6.5.1
Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances
from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment
Modified Randomized Set

Treatment Group : Placebo

Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks
Number of red blood cell (RBC) units transfused	n	26	26	26
	Mean (SD)	2.308 (2.1683)	2.115 (2.2685)	-0.192 (1.3862)
	Median	2.0	2.0	0.0
	Min, Max	0, 7	0, 8	-3, 2
Number of transfusion instances	n	26	26	26
	Mean (SD)	1.423 (1.3319)	1.269 (1.3434)	-0.154 (1.0842)
	Median	1.0	1.0	0.0
	Min, Max	0, 5	0, 5	-3, 1

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.1.1
Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment
Analysis Using ANCOVA Model
Modified Randomized Set

Variable	Statistics	Danicopan (N = 57)	Placebo (N = 26)	Difference in LS Means (Danicopan-Placebo)
Number of red blood cell (RBC) units transfused	LS Mean (SEM)	-1.40 (0.213)	-0.08 (0.316)	-1.32 (0.382)
	95% CI for LS Mean	(-1.83, -0.98)	(-0.71, 0.55)	(-2.08, -0.57)
	P-value			0.0008
Number of transfusion instances	LS Mean (SEM)	-0.89 (0.137)	-0.12 (0.203)	-0.77 (0.246)
	95% CI for LS Mean	(-1.17, -0.62)	(-0.53, 0.28)	(-1.26, -0.28)
	P-value			0.0024

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment

P-value from ANCOVA for treatment comparison.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.4.12.1

Change in the Number of RBC Units Transfused and Transfusion Instances from 24 Weeks Prior to Initiation of Treatment
to Post 24 Weeks of Treatment for Patients Randomized to Danicopan Group
Modified Randomized Set

Variable	Statistics	Prior 24 Weeks	Post 24 Weeks	Change from Prior 24 Weeks to Post 24 Weeks
Number of red blood cell (RBC) units transfused	n	55	55	55
	Mean (SD)	4.0 (4.34)	1.3 (2.95)	-2.7 (4.86)
	95% CI for Mean	(2.81, 5.16)	(0.46, 2.05)	(-4.04, -1.41)
Number of transfusion instances	n	55	55	55
	Mean (SD)	2.4 (2.00)	0.8 (2.22)	-1.5 (2.41)
	95% CI for Mean	(1.84, 2.92)	(0.24, 1.44)	(-2.20, -0.89)

Post 24 weeks : 24 weeks after first dose of Danicopan treatment; Prior 24 Weeks: 24 weeks prior to first dose of Danicopan treatment.

n is number of patients who entered Treatment Period 2.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.2 Transfusionsvermeidung und -bedarf

1.2.2 Transfundierte Erythrozyten-Einheiten und Transfusionsfälle

1.2.2.1 Subgruppenanalysen – Transfundierte Erythrozyten-Einheiten und Transfusionsfälle

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Table GERMANY.2.6.5.2
Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances
from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Sex
Modified Randomized Set

Male

Treatment Group	Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks	
Danicopan	Number of red blood cell (RBC) units transfused	n	23	23	23	
		Mean (SD)	2.1 (2.30)	0.7 (2.00)	-1.4 (2.55)	
		Median	2.0	0.0	-1.0	
		Min, Max	0, 8	0, 7	-8, 4	
	Number of transfusion instances	n	23	23	23	
		Mean (SD)	1.5 (1.62)	0.5 (1.50)	-1.0 (1.65)	
		Median	1.0	0.0	-1.0	
		Min, Max	0, 6	0, 6	-4, 3	
	Placebo	Number of red blood cell (RBC) units transfused	n	9	9	9
			Mean (SD)	1.9 (1.36)	1.4 (1.51)	-0.4 (1.74)
			Median	2.0	2.0	0.0
			Min, Max	0, 4	0, 4	-3, 2
Number of transfusion instances		n	9	9	9	
		Mean (SD)	1.1 (0.78)	0.9 (0.93)	-0.2 (1.20)	
		Median	1.0	1.0	0.0	
		Min, Max	0, 2	0, 2	-2, 1	

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.6.5.2
Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances
from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Sex
Modified Randomized Set

Female

Treatment Group	Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks	
Danicopan	Number of red blood cell (RBC) units transfused	n	34	34	34	
		Mean (SD)	2.0 (2.35)	0.6 (1.28)	-1.3 (2.28)	
		Median	1.0	0.0	-1.0	
		Min, Max	0, 11	0, 5	-11, 2	
	Number of transfusion instances	n	34	34	34	
		Mean (SD)	1.2 (1.12)	0.4 (0.86)	-0.8 (1.04)	
		Median	1.0	0.0	-1.0	
		Min, Max	0, 4	0, 4	-4, 1	
	Placebo	Number of red blood cell (RBC) units transfused	n	17	17	17
			Mean (SD)	2.5 (2.50)	2.5 (2.55)	-0.1 (1.20)
			Median	2.0	2.0	0.0
			Min, Max	0, 7	0, 8	-3, 2
Number of transfusion instances		n	17	17	17	
		Mean (SD)	1.6 (1.54)	1.5 (1.50)	-0.1 (1.05)	
		Median	2.0	1.0	0.0	
		Min, Max	0, 5	0, 5	-3, 1	

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.6.5.3

Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

< 65 Years

Treatment Group	Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks	
Danicopan	Number of red blood cell (RBC) units transfused	n	41	41	41	
		Mean (SD)	1.8 (2.42)	0.7 (1.62)	-1.1 (2.57)	
		Median	1.0	0.0	0.0	
		Min, Max	0, 11	0, 7	-11, 4	
	Number of transfusion instances	n	41	41	41	
		Mean (SD)	1.1 (1.24)	0.4 (1.18)	-0.7 (1.28)	
		Median	1.0	0.0	0.0	
		Min, Max	0, 4	0, 6	-4, 3	
	Placebo	Number of red blood cell (RBC) units transfused	n	20	20	20
			Mean (SD)	2.6 (2.31)	2.3 (2.53)	-0.3 (1.42)
			Median	2.5	1.5	0.0
			Min, Max	0, 7	0, 8	-3, 2
Number of transfusion instances		n	20	20	20	
		Mean (SD)	1.6 (1.43)	1.4 (1.50)	-0.3 (1.16)	
		Median	2.0	1.5	0.0	
		Min, Max	0, 5	0, 5	-3, 1	

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.6.5.3

Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Treatment Group	Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks	
Danicopan	Number of red blood cell (RBC) units transfused	n	16	16	16	
		Mean (SD)	2.6 (1.96)	0.8 (1.57)	-1.9 (1.71)	
		Median	2.0	0.0	-2.0	
		Min, Max	0, 7	0, 5	-5, 2	
	Number of transfusion instances	n	16	16	16	
		Mean (SD)	1.9 (1.44)	0.5 (1.10)	-1.4 (1.26)	
		Median	1.0	0.0	-1.0	
		Min, Max	1, 6	0, 4	-4, 1	
	Placebo	Number of red blood cell (RBC) units transfused	n	6	6	6
			Mean (SD)	1.5 (1.52)	1.7 (1.03)	0.2 (1.33)
			Median	1.5	2.0	0.0
			Min, Max	0, 4	0, 3	-2, 2
Number of transfusion instances		n	6	6	6	
		Mean (SD)	0.8 (0.75)	1.0 (0.63)	0.2 (0.75)	
		Median	1.0	1.0	0.0	
		Min, Max	0, 2	0, 2	-1, 1	

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.6.5.4
Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances
from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Region
Modified Randomized Set

Europe

Treatment Group	Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks	
Danicopan	Number of red blood cell (RBC) units transfused	n	25	25	25	
		Mean (SD)	2.3 (2.70)	1.2 (2.17)	-1.0 (2.95)	
		Median	1.0	0.0	-1.0	
		Min, Max	0, 11	0, 7	-11, 4	
	Number of transfusion instances	n	25	25	25	
		Mean (SD)	1.6 (1.60)	0.8 (1.60)	-0.8 (1.68)	
		Median	1.0	0.0	-1.0	
		Min, Max	0, 6	0, 6	-4, 3	
	Placebo	Number of red blood cell (RBC) units transfused	n	13	13	13
			Mean (SD)	2.8 (2.15)	2.1 (2.06)	-0.8 (1.36)
			Median	3.0	2.0	0.0
			Min, Max	0, 7	0, 6	-3, 1
Number of transfusion instances		n	13	13	13	
		Mean (SD)	1.8 (1.36)	1.2 (1.09)	-0.5 (1.27)	
		Median	2.0	2.0	0.0	
		Min, Max	0, 5	0, 3	-3, 1	

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.6.5.4
Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances
from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Region
Modified Randomized Set

Rest of World

Treatment Group	Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks	
Danicopan	Number of red blood cell (RBC) units transfused	n	32	32	32	
		Mean (SD)	1.8 (1.99)	0.3 (0.72)	-1.6 (1.81)	
		Median	1.5	0.0	-1.0	
		Min, Max	0, 8	0, 3	-8, 0	
	Number of transfusion instances	n	32	32	32	
		Mean (SD)	1.1 (1.06)	0.2 (0.45)	-0.9 (0.95)	
		Median	1.0	0.0	-1.0	
		Min, Max	0, 4	0, 2	-4, 0	
	Placebo	Number of red blood cell (RBC) units transfused	n	13	13	13
			Mean (SD)	1.8 (2.13)	2.2 (2.54)	0.4 (1.19)
			Median	1.0	2.0	0.0
			Min, Max	0, 7	0, 8	-2, 2
Number of transfusion instances		n	13	13	13	
		Mean (SD)	1.1 (1.26)	1.3 (1.60)	0.2 (0.73)	
		Median	1.0	1.0	0.0	
		Min, Max	0, 4	0, 5	-1, 1	

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.6.5.5

Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Treatment Group	Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks	
Danicopan	Number of red blood cell (RBC) units transfused	n	24	24	24	
		Mean (SD)	3.3 (2.57)	1.1 (2.17)	-2.2 (3.20)	
		Median	3.0	0.0	-2.0	
		Min, Max	0, 11	0, 7	-11, 4	
	Number of transfusion instances	n	24	24	24	
		Mean (SD)	2.0 (1.27)	0.8 (1.64)	-1.3 (1.65)	
		Median	2.0	0.0	-1.0	
		Min, Max	0, 4	0, 6	-4, 3	
	Placebo	Number of red blood cell (RBC) units transfused	n	10	10	10
			Mean (SD)	3.7 (2.41)	3.3 (2.83)	-0.4 (1.96)
			Median	3.5	3.5	0.0
			Min, Max	0, 7	0, 8	-3, 2
Number of transfusion instances		n	10	10	10	
		Mean (SD)	2.4 (1.43)	1.9 (1.73)	-0.5 (1.43)	
		Median	2.0	2.0	0.0	
		Min, Max	0, 5	0, 5	-3, 1	

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.6.5.5

Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Treatment Group	Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks	
Danicopan	Number of red blood cell (RBC) units transfused	n	33	33	33	
		Mean (SD)	1.1 (1.57)	0.4 (0.90)	-0.7 (1.23)	
		Median	1.0	0.0	0.0	
		Min, Max	0, 7	0, 4	-4, 2	
	Number of transfusion instances	n	33	33	33	
		Mean (SD)	0.8 (1.16)	0.2 (0.48)	-0.6 (0.93)	
		Median	1.0	0.0	0.0	
		Min, Max	0, 6	0, 2	-4, 1	
	Placebo	Number of red blood cell (RBC) units transfused	n	16	16	16
			Mean (SD)	1.4 (1.50)	1.4 (1.50)	-0.1 (0.93)
			Median	1.5	1.0	0.0
			Min, Max	0, 4	0, 4	-2, 2
Number of transfusion instances		n	16	16	16	
		Mean (SD)	0.8 (0.83)	0.9 (0.89)	0.1 (0.77)	
		Median	1.0	1.0	0.0	
		Min, Max	0, 2	0, 2	-2, 1	

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.6.5.6

Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and \geq 8.5 g/dL)
Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Treatment Group	Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks	
Danicopan	Number of red blood cell (RBC) units transfused	n	35	35	35	
		Mean (SD)	2.9 (2.49)	1.1 (1.92)	-1.8 (2.79)	
		Median	3.0	0.0	-2.0	
		Min, Max	0, 11	0, 7	-11, 4	
	Number of transfusion instances	n	35	35	35	
		Mean (SD)	1.9 (1.42)	0.7 (1.41)	-1.1 (1.54)	
		Median	2.0	0.0	-1.0	
		Min, Max	0, 6	0, 6	-4, 3	
	Placebo	Number of red blood cell (RBC) units transfused	n	15	15	15
			Mean (SD)	2.4 (2.13)	2.4 (2.67)	0.0 (1.36)
			Median	2.0	2.0	0.0
			Min, Max	0, 7	0, 8	-3, 2
Number of transfusion instances		n	15	15	15	
		Mean (SD)	1.5 (1.25)	1.5 (1.60)	0.0 (1.00)	
		Median	1.0	1.0	0.0	
		Min, Max	0, 4	0, 5	-2, 1	

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.6.5.6

Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Treatment Group	Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks	
Danicopan	Number of red blood cell (RBC) units transfused	n	22	22	22	
		Mean (SD)	0.7 (1.04)	0.1 (0.43)	-0.6 (1.18)	
		Median	0.0	0.0	0.0	
		Min, Max	0, 4	0, 2	-4, 2	
	Number of transfusion instances	n	22	22	22	
		Mean (SD)	0.5 (0.60)	0.0 (0.21)	-0.5 (0.67)	
		Median	0.0	0.0	0.0	
		Min, Max	0, 2	0, 1	-2, 1	
	Placebo	Number of red blood cell (RBC) units transfused	n	11	11	11
			Mean (SD)	2.2 (2.32)	1.7 (1.62)	-0.5 (1.44)
			Median	2.0	2.0	0.0
			Min, Max	0, 7	0, 4	-3, 2
Number of transfusion instances		n	11	11	11	
		Mean (SD)	1.4 (1.50)	1.0 (0.89)	-0.4 (1.21)	
		Median	1.0	1.0	0.0	
		Min, Max	0, 5	0, 2	-3, 1	

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.6.5.7

Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Treatment Group	Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks	
Danicopan	Number of red blood cell (RBC) units transfused	n	21	21	21	
		Mean (SD)	2.3 (2.85)	1.3 (2.20)	-1.0 (3.00)	
		Median	1.0	0.0	-1.0	
		Min, Max	0, 11	0, 7	-11, 4	
	Number of transfusion instances	n	21	21	21	
		Mean (SD)	1.7 (1.62)	0.9 (1.59)	-0.8 (1.63)	
		Median	1.0	0.0	-1.0	
		Min, Max	0, 6	0, 6	-4, 3	
	Placebo	Number of red blood cell (RBC) units transfused	n	12	12	12
			Mean (SD)	2.2 (2.48)	1.9 (2.07)	-0.3 (1.29)
			Median	1.5	1.5	0.0
			Min, Max	0, 7	0, 6	-3, 2
Number of transfusion instances		n	12	12	12	
		Mean (SD)	1.3 (1.56)	1.2 (1.11)	-0.2 (1.19)	
		Median	1.0	1.5	0.0	
		Min, Max	0, 5	0, 3	-3, 1	

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.6.5.7

Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Treatment Group	Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks	
Danicopan	Number of red blood cell (RBC) units transfused	n	36	36	36	
		Mean (SD)	1.9 (1.96)	0.3 (0.95)	-1.6 (1.93)	
		Median	1.5	0.0	-1.0	
		Min, Max	0, 8	0, 5	-8, 2	
	Number of transfusion instances	n	36	36	36	
		Mean (SD)	1.1 (1.13)	0.2 (0.72)	-0.9 (1.11)	
		Median	1.0	0.0	-1.0	
		Min, Max	0, 4	0, 4	-4, 1	
	Placebo	Number of red blood cell (RBC) units transfused	n	14	14	14
			Mean (SD)	2.4 (1.95)	2.3 (2.49)	-0.1 (1.51)
			Median	2.0	2.0	0.0
			Min, Max	0, 7	0, 8	-3, 2
Number of transfusion instances		n	14	14	14	
		Mean (SD)	1.5 (1.16)	1.4 (1.55)	-0.1 (1.03)	
		Median	1.5	1.0	0.0	
		Min, Max	0, 4	0, 5	-2, 1	

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.6.5.8

Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Patients Enrolled from Japan Yes

Treatment Group	Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks	
Danicopan	Number of red blood cell (RBC) units transfused	n	8	8	8	
		Mean (SD)	4.3 (1.98)	0.5 (0.93)	-3.8 (1.98)	
		Median	4.0	0.0	-4.0	
		Min, Max	2, 8	0, 2	-8, -2	
	Number of transfusion instances	n	8	8	8	
		Mean (SD)	2.1 (0.99)	0.3 (0.46)	-1.9 (0.99)	
		Median	2.0	0.0	-2.0	
		Min, Max	1, 4	0, 1	-4, -1	
	Placebo	Number of red blood cell (RBC) units transfused	n	4	4	4
			Mean (SD)	2.0 (1.63)	1.5 (1.00)	-0.5 (1.00)
			Median	2.0	2.0	0.0
			Min, Max	0, 4	0, 2	-2, 0
Number of transfusion instances		n	4	4	4	
		Mean (SD)	1.0 (0.82)	0.8 (0.50)	-0.3 (0.50)	
		Median	1.0	1.0	0.0	
		Min, Max	0, 2	0, 1	-1, 0	

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.6.5.8

Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Patients Enrolled from Japan No

Treatment Group	Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks	
Danicopan	Number of red blood cell (RBC) units transfused	n	49	49	49	
		Mean (SD)	1.7 (2.17)	0.7 (1.68)	-1.0 (2.21)	
		Median	1.0	0.0	-1.0	
		Min, Max	0, 11	0, 7	-11, 4	
	Number of transfusion instances	n	49	49	49	
		Mean (SD)	1.2 (1.35)	0.5 (1.23)	-0.7 (1.29)	
		Median	1.0	0.0	-1.0	
		Min, Max	0, 6	0, 6	-4, 3	
	Placebo	Number of red blood cell (RBC) units transfused	n	22	22	22
			Mean (SD)	2.4 (2.28)	2.2 (2.43)	-0.1 (1.46)
			Median	2.0	1.5	0.0
			Min, Max	0, 7	0, 8	-3, 2
Number of transfusion instances		n	22	22	22	
		Mean (SD)	1.5 (1.41)	1.4 (1.43)	-0.1 (1.17)	
		Median	1.5	1.5	0.0	
		Min, Max	0, 5	0, 5	-3, 1	

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.6.5.9

Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Race
Modified Randomized Set

Asian

Treatment Group	Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks	
Danicopan	Number of red blood cell (RBC) units transfused	n	22	22	22	
		Mean (SD)	2.1 (2.18)	0.2 (0.59)	-1.9 (2.00)	
		Median	2.0	0.0	-2.0	
		Min, Max	0, 8	0, 2	-8, 0	
	Number of transfusion instances	n	22	22	22	
		Mean (SD)	1.2 (1.10)	0.1 (0.29)	-1.1 (1.02)	
		Median	1.0	0.0	-1.0	
		Min, Max	0, 4	0, 1	-4, 0	
	Placebo	Number of red blood cell (RBC) units transfused	n	10	10	10
			Mean (SD)	2.2 (2.25)	2.4 (2.80)	0.2 (1.23)
			Median	2.0	2.0	0.0
			Min, Max	0, 7	0, 8	-2, 2
Number of transfusion instances		n	10	10	10	
		Mean (SD)	1.3 (1.34)	1.4 (1.78)	0.1 (0.74)	
		Median	1.0	1.0	0.0	
		Min, Max	0, 4	0, 5	-1, 1	

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.6.5.9

Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Race
Modified Randomized Set

White

Treatment Group	Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks	
Danicopan	Number of red blood cell (RBC) units transfused	n	28	28	28	
		Mean (SD)	2.2 (2.63)	1.2 (2.10)	-1.0 (2.80)	
		Median	1.5	0.0	-0.5	
		Min, Max	0, 11	0, 7	-11, 4	
	Number of transfusion instances	n	28	28	28	
		Mean (SD)	1.6 (1.60)	0.8 (1.54)	-0.8 (1.60)	
		Median	1.0	0.0	-0.5	
		Min, Max	0, 6	0, 6	-4, 3	
	Placebo	Number of red blood cell (RBC) units transfused	n	11	11	11
			Mean (SD)	1.9 (1.45)	1.5 (1.69)	-0.4 (1.50)
			Median	2.0	1.0	0.0
			Min, Max	0, 4	0, 4	-3, 2
Number of transfusion instances		n	11	11	11	
		Mean (SD)	1.3 (0.90)	1.0 (1.00)	-0.3 (1.19)	
		Median	2.0	1.0	0.0	
		Min, Max	0, 2	0, 2	-2, 1	

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.6.5.9

Summary of Actual Values and Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Race
Modified Randomized Set

Others or Unknown

Treatment Group	Variable	Statistics	Prior 12 weeks	Post 12 weeks	Change from Prior 12 weeks to Post 12 weeks	
Danicopan	Number of red blood cell (RBC) units transfused	n	7	7	7	
		Mean (SD)	1.1 (1.07)	0.1 (0.38)	-1.0 (1.15)	
		Median	1.0	0.0	-1.0	
		Min, Max	0, 3	0, 1	-3, 0	
	Number of transfusion instances	n	7	7	7	
		Mean (SD)	0.9 (0.69)	0.1 (0.38)	-0.7 (0.76)	
		Median	1.0	0.0	-1.0	
		Min, Max	0, 2	0, 1	-2, 0	
	Placebo	Number of red blood cell (RBC) units transfused	n	5	5	5
			Mean (SD)	3.4 (3.29)	2.8 (2.39)	-0.6 (1.52)
			Median	4.0	3.0	0.0
			Min, Max	0, 7	0, 6	-3, 1
Number of transfusion instances		n	5	5	5	
		Mean (SD)	2.0 (2.12)	1.6 (1.14)	-0.4 (1.52)	
		Median	2.0	2.0	0.0	
		Min, Max	0, 5	0, 3	-3, 1	

Post 12 weeks: 12 weeks after first dose of treatment; Prior 12 Weeks: 12 weeks prior to first dose of treatment.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.1.2
Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Sex
Analysis Using ANCOVA Model
Modified Randomized Set

Male

Variable	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference in LS Means (Danicopan-Placebo)
Number of red blood cell (RBC) units transfused	LS Mean (SEM)	-1.34 (0.382)	-0.57 (0.611)	-0.77 (0.721)
	95% CI for LS Mean	(-2.12, -0.56)	(-1.82, 0.68)	(-2.24, 0.70)
	P-value			0.2943
Number of transfusion instances	LS Mean (SEM)	-0.93 (0.268)	-0.40 (0.429)	-0.52 (0.508)
	95% CI for LS Mean	(-1.48, -0.38)	(-1.28, 0.47)	(-1.56, 0.52)
	P-value			0.3111

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment
P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional sex variable and sex by treatment interaction term.
Source: ADaM.ADSL, ADaM.ADEFF
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Table GERMANY.2.3.1.2
Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of
Treatment to Post 12 Weeks of Treatment by Sex
Analysis Using ANCOVA Model
Modified Randomized Set

Female

Variable	Statistics	Danicopan (N = 34)	Placebo (N = 17)	Difference in LS Means (Danicopan-Placebo)
Number of red blood cell (RBC) units transfused	LS Mean (SEM)	-1.43 (0.253)	0.15 (0.359)	-1.58 (0.440)
	95% CI for LS Mean	(-1.94, -0.92)	(-0.57, 0.87)	(-2.47, -0.69)
	P-value			0.0008
Number of transfusion instances	LS Mean (SEM)	-0.85 (0.151)	0.00 (0.214)	-0.85 (0.263)
	95% CI for LS Mean	(-1.16, -0.55)	(-0.43, 0.43)	(-1.38, -0.32)
	P-value			0.0022

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment

P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional sex variable and sex by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.1.2
Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of
Treatment to Post 12 Weeks of Treatment by Sex
Analysis Using ANCOVA Model
Modified Randomized Set

Interaction p-value

Variable	Interaction p-value
Number of red blood cell (RBC) units transfused	0.3129
Number of transfusion instances	0.5441

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment

P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional sex variable and sex by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.1.3

Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Age at Informed Consent (< 65 and ≥ 65 Years)

Analysis Using ANCOVA Model
Modified Randomized Set

< 65 Years

Variable	Statistics	Danicopan (N = 41)	Placebo (N = 20)	Difference in LS Means (Danicopan-Placebo)
Number of red blood cell (RBC) units transfused	LS Mean (SEM)	-1.30 (0.274)	0.01 (0.393)	-1.30 (0.482)
	95% CI for LS Mean	(-1.84, -0.75)	(-0.78, 0.80)	(-2.27, -0.34)
	P-value			0.0089
Number of transfusion instances	LS Mean (SEM)	-0.73 (0.172)	-0.09 (0.248)	-0.64 (0.304)
	95% CI for LS Mean	(-1.08, -0.39)	(-0.59, 0.40)	(-1.25, -0.03)
	P-value			0.0393

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
 Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment
 P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional age variable and age by treatment interaction term.
 Source: ADaM.ADSL, ADaM.ADEFF
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Table GERMANY.2.3.1.3

Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Age at Informed Consent (< 65 and ≥ 65 Years)

Analysis Using ANCOVA Model

Modified Randomized Set

≥ 65 Years

Variable	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference in LS Means (Danicopan-Placebo)
Number of red blood cell (RBC) units transfused	LS Mean (SEM)	-1.70 (0.316)	-0.30 (0.526)	-1.40 (0.623)
	95% CI for LS Mean	(-2.36, -1.04)	(-1.40, 0.80)	(-2.70, -0.09)
	P-value			0.0371
Number of transfusion instances	LS Mean (SEM)	-1.26 (0.224)	-0.32 (0.378)	-0.94 (0.452)
	95% CI for LS Mean	(-1.73, -0.79)	(-1.11, 0.48)	(-1.89, 0.00)
	P-value			0.0508

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment

P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional age variable and age by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.1.3

Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Age at Informed Consent (< 65 and ≥ 65 Years)

Analysis Using ANCOVA Model
Modified Randomized Set

Interaction p-value

Variable	Interaction p-value
Number of red blood cell (RBC) units transfused	0.9475
Number of transfusion instances	0.5035

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment
P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional age variable and age by treatment interaction term.
Source: ADaM.ADSL, ADaM.ADEFF
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Table GERMANY.2.3.1.4
Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Region
Analysis Using ANCOVA Model
Modified Randomized Set

Europe

Variable	Statistics	Danicopan (N = 25)	Placebo (N = 13)	Difference in LS Means (Danicopan-Placebo)
Number of red blood cell (RBC) units transfused	LS Mean (SEM)	-1.17 (0.393)	-0.53 (0.546)	-0.64 (0.675)
	95% CI for LS Mean	(-1.96, -0.37)	(-1.64, 0.58)	(-2.01, 0.73)
	P-value			0.3517
Number of transfusion instances	LS Mean (SEM)	-0.83 (0.262)	-0.49 (0.363)	-0.34 (0.448)
	95% CI for LS Mean	(-1.36, -0.29)	(-1.23, 0.25)	(-1.24, 0.57)
	P-value			0.4598

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment
P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional region variable and region by treatment interaction term.
Source: ADaM.ADSL, ADaM.ADEFF
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Table GERMANY.2.3.1.4
Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of
Treatment to Post 12 Weeks of Treatment by Region
Analysis Using ANCOVA Model
Modified Randomized Set

Rest of World

Variable	Statistics	Danicopan (N = 32)	Placebo (N = 13)	Difference in LS Means (Danicopan-Placebo)
Number of red blood cell (RBC) units transfused	LS Mean (SEM)	-1.58 (0.213)	0.35 (0.335)	-1.94 (0.397)
	95% CI for LS Mean	(-2.01, -1.15)	(-0.32, 1.03)	(-2.74, -1.13)
	P-value			<.0001
Number of transfusion instances	LS Mean (SEM)	-0.94 (0.128)	0.23 (0.201)	-1.16 (0.239)
	95% CI for LS Mean	(-1.19, -0.68)	(-0.18, 0.63)	(-1.64, -0.68)
	P-value			<.0001

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment

P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional region variable and region by treatment interaction term.

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Table GERMANY.2.3.1.4
Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Region
Analysis Using ANCOVA Model
Modified Randomized Set

Interaction p-value

Variable	Interaction p-value
Number of red blood cell (RBC) units transfused	0.0856
Number of transfusion instances	0.0933

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment
P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional region variable and region by treatment interaction term.
Source: ADaM.ADSL, ADaM.ADEFF
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Table GERMANY.2.3.1.5

Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using ANCOVA Model
Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Variable	Statistics	Danicopan (N = 24)	Placebo (N = 10)	Difference in LS Means (Danicopan-Placebo)
Number of red blood cell (RBC) units transfused	LS Mean (SEM)	-2.29 (0.471)	-0.21 (0.730)	-2.07 (0.870)
	95% CI for LS Mean	(-3.25, -1.33)	(-1.70, 1.28)	(-3.85, -0.30)
	P-value			0.0235
Number of transfusion instances	LS Mean (SEM)	-1.30 (0.309)	-0.39 (0.481)	-0.90 (0.573)
	95% CI for LS Mean	(-1.93, -0.66)	(-1.37, 0.59)	(-2.07, 0.27)
	P-value			0.1252

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment
P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional transfusion history variable and transfusion history by treatment interaction term.
Source: ADaM.ADSL, ADaM.ADEFF
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Table GERMANY.2.3.1.5

Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using ANCOVA Model
Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Variable	Statistics	Danicopan (N = 33)	Placebo (N = 16)	Difference in LS Means (Danicopan-Placebo)
Number of red blood cell (RBC) units transfused	LS Mean (SEM)	-0.78 (0.146)	0.06 (0.210)	-0.84 (0.257)
	95% CI for LS Mean	(-1.08, -0.49)	(-0.37, 0.48)	(-1.36, -0.32)
	P-value			0.0020
Number of transfusion instances	LS Mean (SEM)	-0.60 (0.093)	0.06 (0.134)	-0.66 (0.164)
	95% CI for LS Mean	(-0.79, -0.42)	(-0.21, 0.33)	(-0.99, -0.34)
	P-value			0.0002

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment
P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional transfusion history variable and transfusion history by treatment interaction term.
Source: ADaM.ADSL, ADaM.ADEFF
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Table GERMANY.2.3.1.5

Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using ANCOVA Model
Modified Randomized Set

Interaction p-value

Variable	Interaction p-value
Number of red blood cell (RBC) units transfused	0.1401
Number of transfusion instances	0.5839

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment
P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional transfusion history variable and transfusion history by treatment interaction term.
Source: ADaM.ADSL, ADaM.ADEFF
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Table GERMANY.2.3.1.6

Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)

Analysis Using ANCOVA Model
Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Variable	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference in LS Means (Danicopan-Placebo)
Number of red blood cell (RBC) units transfused	LS Mean (SEM)	-1.74 (0.336)	-0.21 (0.515)	-1.53 (0.616)
	95% CI for LS Mean	(-2.41, -1.06)	(-1.25, 0.82)	(-2.77, -0.29)
	P-value			0.0168
Number of transfusion instances	LS Mean (SEM)	-1.09 (0.215)	-0.12 (0.330)	-0.97 (0.396)
	95% CI for LS Mean	(-1.52, -0.66)	(-0.79, 0.54)	(-1.76, -0.17)
	P-value			0.0184

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
 Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment
 P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.
 Source: ADaM.ADSL, ADaM.ADEFF
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Table GERMANY.2.3.1.6

Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)

Analysis Using ANCOVA Model
Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Variable	Statistics	Danicopan (N = 22)	Placebo (N = 11)	Difference in LS Means (Danicopan-Placebo)
Number of red blood cell (RBC) units transfused	LS Mean (SEM)	-0.91 (0.178)	0.18 (0.261)	-1.08 (0.329)
	95% CI for LS Mean	(-1.27, -0.54)	(-0.36, 0.71)	(-1.76, -0.41)
	P-value			0.0026
Number of transfusion instances	LS Mean (SEM)	-0.67 (0.107)	0.07 (0.155)	-0.74 (0.195)
	95% CI for LS Mean	(-0.89, -0.45)	(-0.25, 0.39)	(-1.14, -0.34)
	P-value			0.0007

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
 Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment
 P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.
 Source: ADaM.ADSL, ADaM.ADEFF
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Table GERMANY.2.3.1.6

Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)

Analysis Using ANCOVA Model
Modified Randomized Set

Interaction p-value

Variable	Interaction p-value
Number of red blood cell (RBC) units transfused	0.5684
Number of transfusion instances	0.4509

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment
P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.
Source: ADaM.ADSL, ADaM.ADEFF
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Table GERMANY.2.3.1.7

Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Analysis Using ANCOVA Model Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Variable	Statistics	Danicopan (N = 21)	Placebo (N = 12)	Difference in LS Means (Danicopan-Placebo)
Number of red blood cell (RBC) units transfused	LS Mean (SEM)	-0.96 (0.418)	-0.32 (0.553)	-0.65 (0.694)
	95% CI for LS Mean	(-1.82, -0.11)	(-1.45, 0.81)	(-2.06, 0.77)
	P-value			0.3584
Number of transfusion instances	LS Mean (SEM)	-0.75 (0.273)	-0.28 (0.361)	-0.47 (0.454)
	95% CI for LS Mean	(-1.30, -0.19)	(-1.02, 0.46)	(-1.39, 0.46)
	P-value			0.3122

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
 Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment
 P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.
 Source: ADaM.ADSL, ADaM.ADEFF
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Table GERMANY.2.3.1.7

Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using ANCOVA Model
Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Variable	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference in LS Means (Danicopan-Placebo)
Number of red blood cell (RBC) units transfused	LS Mean (SEM)	-1.66 (0.228)	0.11 (0.367)	-1.77 (0.433)
	95% CI for LS Mean	(-2.11, -1.20)	(-0.62, 0.85)	(-2.64, -0.90)
	P-value			0.0002
Number of transfusion instances	LS Mean (SEM)	-0.97 (0.149)	0.00 (0.240)	-0.97 (0.284)
	95% CI for LS Mean	(-1.27, -0.67)	(-0.48, 0.48)	(-1.55, -0.40)
	P-value			0.0012

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment

P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.1.7

Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using ANCOVA Model
Modified Randomized Set

Interaction p-value

Variable	Interaction p-value
Number of red blood cell (RBC) units transfused	0.1472
Number of transfusion instances	0.3152

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment

P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.1.8

Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment
to Post 12 Weeks of Treatment by the Randomization Stratification Factors of Japanese Patients
(Patients Enrolled from Japan [Yes/No])
Analysis Using ANCOVA Model
Modified Randomized Set

Patients Enrolled from Japan Yes

Variable	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference in LS Means (Danicopan-Placebo)
Number of red blood cell (RBC) units transfused	LS Mean (SEM)	-3.15 (0.345)	-1.70 (0.515)	-1.44 (0.662)
	95% CI for LS Mean	(-3.93, -2.37)	(-2.87, -0.54)	(-2.94, 0.05)
	P-value			0.0571
Number of transfusion instances	LS Mean (SEM)	-1.57 (0.173)	-0.85 (0.258)	-0.72 (0.331)
	95% CI for LS Mean	(-1.96, -1.18)	(-1.43, -0.27)	(-1.47, 0.03)
	P-value			0.0571

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment

P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Run Date: 2024-04-26T15:50:44

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Table GERMANY.2.3.1.8

Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using ANCOVA Model
Modified Randomized Set

Patients Enrolled from Japan No

Variable	Statistics	Danicopan (N = 49)	Placebo (N = 22)	Difference in LS Means (Danicopan-Placebo)
Number of red blood cell (RBC) units transfused	LS Mean (SEM)	-1.07 (0.236)	0.12 (0.354)	-1.19 (0.428)
	95% CI for LS Mean	(-1.54, -0.60)	(-0.59, 0.82)	(-2.04, -0.33)
	P-value			0.0071
Number of transfusion instances	LS Mean (SEM)	-0.76 (0.155)	-0.04 (0.231)	-0.72 (0.279)
	95% CI for LS Mean	(-1.07, -0.45)	(-0.50, 0.42)	(-1.27, -0.16)
	P-value			0.0122

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment
P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional Japanese patients variable and Japanese patients by treatment interaction term.
Source: ADaM.ADSL, ADaM.ADEFF
Run Date: 2024-04-26T15:50:44
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Table GERMANY.2.3.1.8

Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using ANCOVA Model
Modified Randomized Set

Interaction p-value

Variable	Interaction p-value
Number of red blood cell (RBC) units transfused	0.4609
Number of transfusion instances	0.6215

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment
P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional Japanese patients variable and Japanese patients by treatment interaction term.
Source: ADaM.ADSL, ADaM.ADEFF
Run Date: 2024-04-26T15:50:44
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Table GERMANY.2.3.1.9
Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Race
Analysis Using ANCOVA Model
Modified Randomized Set

Asian

Variable	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference in LS Means (Danicopan-Placebo)
Number of red blood cell (RBC) units transfused	LS Mean (SEM)	-1.93 (0.281)	0.24 (0.417)	-2.17 (0.504)
	95% CI for LS Mean	(-2.50, -1.35)	(-0.61, 1.10)	(-3.20, -1.14)
	P-value			0.0002
Number of transfusion instances	LS Mean (SEM)	-1.11 (0.168)	0.14 (0.250)	-1.25 (0.301)
	95% CI for LS Mean	(-1.45, -0.76)	(-0.37, 0.65)	(-1.86, -0.63)
	P-value			0.0003

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment
P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional race variable and race by treatment interaction term.
Source: ADaM.ADSL, ADaM.ADEFF
Run Date: 2024-04-26T15:50:44
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Table GERMANY.2.3.1.9

Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment
to Post 12 Weeks of Treatment by Race
Analysis Using ANCOVA Model
Modified Randomized Set

White

Variable	Statistics	Danicopan (N = 28)	Placebo (N = 11)	Difference in LS Means (Danicopan-Placebo)
Number of red blood cell (RBC) units transfused	LS Mean (SEM)	-0.94 (0.359)	-0.52 (0.574)	-0.42 (0.677)
	95% CI for LS Mean	(-1.67, -0.21)	(-1.68, 0.64)	(-1.79, 0.95)
	P-value			0.5393
Number of transfusion instances	LS Mean (SEM)	-0.70 (0.243)	-0.39 (0.388)	-0.31 (0.459)
	95% CI for LS Mean	(-1.20, -0.21)	(-1.18, 0.39)	(-1.24, 0.62)
	P-value			0.5035

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.

Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment

P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional race variable and race by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:50:44

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Table GERMANY.2.3.1.9
Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment to Post 12 Weeks of Treatment by Race
Analysis Using ANCOVA Model
Modified Randomized Set

Others or Unknown

Variable	Statistics	Danicopan (N = 7)	Placebo (N = 5)	Difference in LS Means (Danicopan-Placebo)
Number of red blood cell (RBC) units transfused	LS Mean (SEM)	-1.41 (0.371)	-0.03 (0.448)	-1.38 (0.617)
	95% CI for LS Mean	(-2.25, -0.57)	(-1.04, 0.99)	(-2.78, 0.01)
	P-value			0.0517
Number of transfusion instances	LS Mean (SEM)	-1.03 (0.248)	0.04 (0.298)	-1.07 (0.403)
	95% CI for LS Mean	(-1.59, -0.47)	(-0.64, 0.71)	(-1.98, -0.15)
	P-value			0.0268

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment
P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional race variable and race by treatment interaction term.
Source: ADaM.ADSL, ADaM.ADEFF
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Table GERMANY.2.3.1.9
Change in the Number of RBC Units Transfused and Transfusion Instances from 12 Weeks Prior to Initiation of Treatment
to Post 12 Weeks of Treatment by Race
Analysis Using ANCOVA Model
Modified Randomized Set

Interaction p-value

Variable	Interaction p-value
Number of red blood cell (RBC) units transfused	0.1057
Number of transfusion instances	0.2206

ANCOVA=Analysis of Covariance Model; LS=Least squares; CI=Confidence Interval; SEM=Standard error of the mean.
Estimates are based on ANCOVA that includes treatment group and transfusion units/instances from 12-Week prior initiation of treatment
P-value from ANCOVA for treatment comparison. Interaction test p-value is from the same ANCOVA model with additional race variable and race by treatment
interaction term.
Source: ADaM.ADSL, ADaM.ADEFF
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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.3 Veränderung des LDH-Wertes gegenüber Baseline

Post-hoc Analysen Studie ALPHA

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Table GERMANY.2.6.8.1
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	56		25		81	
Mean (SD)	304.00 (123.600)		279.56 (84.666)		296.45 (113.059)	
Median	261.00		258.50		260.50	
Min, Max	140.0, 809.0		139.0, 506.0		139.0, 809.0	
Week 1						
n	51	50	25	24		
Mean (SD)	246.94 (81.129)	-65.73 (72.752)	301.20 (211.883)	-20.15 (83.416)		
Median	226.00	-48.00	247.00	-12.00		
Min, Max	137.0, 532.0	-295.5, 87.0	128.0, 1260.0	-332.0, 155.0		
Week 2						
n	57	56	26	25		
Mean (SD)	248.11 (121.233)	-68.48 (79.063)	328.88 (272.342)	12.08 (211.636)		
Median	227.00	-50.00	260.00	-7.00		
Min, Max	117.0, 953.0	-307.5, 66.5	144.0, 1260.0	-345.0, 955.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

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Table GERMANY.2.6.8.1
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	54	53	24	23		
Mean (SD)	231.54 (75.696)	-76.63 (83.966)	303.04 (219.084)	-10.20 (78.309)		
Median	228.50	-54.00	261.00	-10.00		
Min, Max	121.0, 540.0	-398.5, 24.0	123.0, 1260.0	-185.0, 193.0		
Week 4						
n	56	55	22	21		
Mean (SD)	264.52 (116.641)	-49.50 (78.836)	311.45 (221.971)	2.40 (34.797)		
Median	258.50	-36.00	270.00	10.00		
Min, Max	108.0, 847.0	-259.5, 114.0	123.0, 1260.0	-82.5, 76.5		
Week 6						
n	54	53	25	24		
Mean (SD)	273.15 (117.113)	-37.69 (72.875)	331.60 (239.259)	22.79 (140.387)		
Median	254.50	-26.50	258.00	1.25		
Min, Max	124.0, 794.0	-238.5, 142.0	130.0, 1260.0	-167.0, 603.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Source: ADaM.ADSL, ADaM.ADLB
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Table GERMANY.2.6.8.1
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	54	53	24	23		
Mean (SD)	283.78 (104.175)	-23.33 (86.341)	334.42 (226.148)	28.48 (99.644)		
Median	257.00	-26.50	271.50	10.00		
Min, Max	145.0, 629.0	-337.5, 255.5	122.0, 1260.0	-113.5, 432.0		
Week 10						
n	51	50	25	24		
Mean (SD)	263.06 (73.934)	-30.10 (71.487)	308.36 (213.925)	-1.42 (74.086)		
Median	251.00	-17.75	256.00	6.75		
Min, Max	148.0, 589.0	-220.0, 82.5	155.0, 1260.0	-137.0, 191.5		
Week 12						
n	52	51	25	24		
Mean (SD)	265.40 (70.963)	-35.16 (93.173)	310.08 (210.685)	0.38 (38.985)		
Median	264.00	-13.50	251.00	2.25		
Min, Max	135.0, 519.0	-379.5, 99.5	128.0, 1260.0	-84.5, 80.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Source: ADaM.ADSL, ADaM.ADLB
Run Date: 2024-04-26T15:50:17
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Table GERMANY.2.6.8.1
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	53	52	23	22		
Mean (SD)	272.02 (119.215)	-30.51 (136.457)	238.78 (141.607)	-50.98 (61.312)		
Median	251.00	-11.50	219.00	-28.50		
Min, Max	129.0, 882.0	-496.5, 627.5	122.0, 854.0	-196.0, 27.5		
Week 14						
n	53	52	23	22		
Mean (SD)	273.15 (94.822)	-28.48 (112.305)	234.13 (75.690)	-40.82 (60.348)		
Median	260.00	-16.25	221.00	-13.75		
Min, Max	135.0, 587.0	-442.5, 214.5	118.0, 501.0	-189.0, 22.5		
Week 15						
n	51	50	22	21		
Mean (SD)	268.61 (93.410)	-35.82 (111.739)	219.59 (52.576)	-47.93 (56.159)		
Median	250.00	-22.75	220.00	-26.00		
Min, Max	134.0, 627.0	-486.5, 283.5	123.0, 361.0	-168.0, 29.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Source: ADaM.ADSL, ADaM.ADLB
Run Date: 2024-04-26T15:50:17
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Table GERMANY.2.6.8.1
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	54	53	24	23		
Mean (SD)	285.06 (104.986)	-15.83 (106.004)	246.17 (70.931)	-22.87 (84.003)		
Median	268.50	-11.50	243.00	-14.00		
Min, Max	144.0, 639.0	-258.5, 332.0	121.0, 439.0	-182.0, 252.0		
Week 18						
n	54	53	23	22		
Mean (SD)	266.93 (79.907)	-33.58 (87.634)	260.26 (71.334)	-6.89 (56.839)		
Median	261.00	-8.00	254.00	-6.00		
Min, Max	159.0, 568.0	-297.5, 113.5	141.0, 461.0	-111.0, 93.5		
Week 20						
n	49	48	24	23		
Mean (SD)	282.00 (109.227)	-28.13 (83.034)	266.04 (79.666)	-3.43 (73.443)		
Median	261.00	-24.75	254.50	-1.00		
Min, Max	157.0, 725.0	-243.5, 235.5	137.0, 535.0	-127.0, 229.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.8.1
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	54	53	24	23		
Mean (SD)	267.80 (114.248)	-37.39 (81.113)	265.58 (58.961)	-3.22 (75.021)		
Median	255.50	-20.00	260.00	15.00		
Min, Max	108.0, 906.0	-307.0, 171.5	169.0, 416.0	-180.0, 110.0		
Week 24						
n	52	51	23	22		
Mean (SD)	276.38 (92.084)	-30.58 (106.379)	280.65 (68.905)	5.86 (80.843)		
Median	268.50	-18.00	264.00	28.00		
Min, Max	130.0, 573.0	-491.5, 142.0	183.0, 442.0	-125.0, 136.0		
Week 28						
n	51	51	23	22	74	73
Mean (SD)	272.00 (94.067)	-30.10 (111.392)	279.00 (67.597)	4.77 (83.893)	274.18 (86.304)	-19.59 (104.543)
Median	257.00	-19.00	286.00	14.00	267.00	-10.00
Min, Max	134.0, 632.0	-467.5, 370.5	173.0, 433.0	-161.0, 143.5	134.0, 632.0	-467.5, 370.5

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.1
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	48	47	22	21	70	68
Mean (SD)	278.17 (87.493)	-32.68 (91.720)	273.45 (78.844)	-4.21 (94.206)	276.69 (84.327)	-23.89 (92.739)
Median	253.00	-7.00	254.50	9.00	253.00	-6.00
Min, Max	150.0, 512.0	-331.5, 101.0	154.0, 421.0	-167.0, 153.5	150.0, 512.0	-331.5, 153.5
Week 36						
n	47	46	20	19	67	65
Mean (SD)	269.79 (100.575)	-18.38 (104.097)	281.95 (80.772)	14.00 (62.652)	273.42 (94.656)	-8.91 (94.570)
Median	256.00	-21.25	266.50	-2.00	256.00	-14.50
Min, Max	131.0, 804.0	-229.5, 470.0	166.0, 478.0	-62.5, 172.0	131.0, 804.0	-229.5, 470.0
Week 40						
n	46	45	21	20	67	65
Mean (SD)	283.30 (83.549)	-17.71 (103.972)	271.57 (68.434)	-7.68 (75.120)	279.63 (78.795)	-14.62 (95.546)
Median	270.00	-8.00	265.00	-12.75	267.00	-8.00
Min, Max	170.0, 623.0	-395.5, 209.0	170.0, 394.0	-164.0, 120.0	170.0, 623.0	-395.5, 209.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.1
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	43	42	20	19	63	61
Mean (SD)	262.12 (57.033)	-38.73 (87.442)	264.30 (58.144)	-12.11 (74.907)	262.81 (56.926)	-30.44 (84.040)
Median	269.00	-25.00	257.00	17.00	268.00	-19.50
Min, Max	149.0, 394.0	-443.5, 93.0	164.0, 373.0	-180.0, 81.0	149.0, 394.0	-443.5, 93.0
Week 48						
n	36	36	20	19	56	55
Mean (SD)	283.33 (72.543)	-7.36 (79.494)	273.45 (68.547)	-5.89 (70.121)	279.80 (70.675)	-6.85 (75.732)
Median	277.00	-8.50	270.50	7.50	275.50	-8.50
Min, Max	157.0, 455.0	-198.5, 230.0	170.0, 413.0	-164.0, 125.5	157.0, 455.0	-198.5, 230.0
Week 52						
n	35	35	19	18	54	53
Mean (SD)	265.26 (61.360)	-35.87 (90.360)	269.00 (67.878)	-12.47 (86.286)	266.57 (63.114)	-27.92 (88.870)
Median	258.00	-24.00	256.00	7.00	257.00	-23.00
Min, Max	155.0, 387.0	-436.5, 82.5	175.0, 455.0	-189.0, 149.0	155.0, 455.0	-436.5, 149.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Run Date: 2024-04-26T15:50:17
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.1
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	36	36	18	17	54	53
Mean (SD)	296.50 (94.985)	-5.65 (114.777)	299.61 (110.519)	17.15 (112.371)	297.54 (99.388)	1.66 (113.436)
Median	282.50	-5.25	262.50	24.00	281.00	-3.50
Min, Max	153.0, 635.0	-329.5, 339.0	186.0, 673.0	-149.0, 339.0	153.0, 673.0	-329.5, 339.0
Week 60						
n	31	31	16	15	47	46
Mean (SD)	264.39 (65.310)	-25.00 (80.503)	276.31 (74.708)	-4.23 (73.115)	268.45 (68.076)	-18.23 (77.978)
Median	261.00	-19.00	248.00	6.50	259.00	-9.75
Min, Max	161.0, 444.0	-199.5, 204.0	166.0, 444.0	-151.0, 110.0	161.0, 444.0	-199.5, 204.0
Week 64						
n	28	28	14	13	42	41
Mean (SD)	277.11 (57.363)	-16.91 (73.984)	263.50 (77.581)	-11.31 (64.909)	272.57 (64.168)	-15.13 (70.468)
Median	283.50	-14.75	233.50	-14.00	265.00	-14.50
Min, Max	151.0, 366.0	-227.5, 84.0	167.0, 421.0	-131.5, 87.0	151.0, 421.0	-227.5, 87.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.1
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	23	23	12	12	35	35
Mean (SD)	270.35 (60.108)	-8.65 (73.609)	238.50 (51.125)	-20.83 (74.576)	259.43 (58.469)	-12.83 (73.073)
Median	278.00	3.50	234.50	1.00	273.00	3.50
Min, Max	137.0, 412.0	-192.0, 99.5	162.0, 343.0	-131.5, 85.0	137.0, 412.0	-192.0, 99.5
Week 72						
n	19	19	11	11	30	30
Mean (SD)	256.79 (61.610)	-18.08 (82.404)	236.00 (41.981)	-20.77 (77.869)	249.17 (55.385)	-19.07 (79.419)
Median	266.00	-3.50	245.00	-16.00	255.00	-6.50
Min, Max	142.0, 403.0	-206.5, 78.0	162.0, 307.0	-138.0, 120.0	142.0, 403.0	-206.5, 120.0
Week 80						
n	17	17	8	8	25	25
Mean (SD)	277.06 (70.692)	-2.91 (92.079)	243.13 (72.292)	-13.13 (39.669)	266.20 (71.532)	-6.18 (78.326)
Median	279.00	-6.00	240.50	-15.00	272.00	-12.50
Min, Max	153.0, 449.0	-162.5, 215.5	171.0, 389.0	-72.5, 52.0	153.0, 449.0	-162.5, 215.5

Baseline is defined as the average of all available assessments prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.1
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	11	11	7	7	18	18
Mean (SD)	305.55 (93.025)	46.32 (106.489)	242.29 (28.022)	5.36 (50.576)	280.94 (79.841)	30.39 (89.418)
Median	292.00	21.50	246.00	9.00	267.00	16.00
Min, Max	176.0, 529.0	-91.0, 329.0	211.0, 274.0	-93.5, 59.0	176.0, 529.0	-93.5, 329.0
Week 96						
n	7	7	6	6	13	13
Mean (SD)	250.29 (58.100)	-18.86 (53.427)	242.67 (64.590)	-3.75 (51.607)	246.77 (58.666)	-11.88 (50.974)
Median	246.00	-10.50	228.50	-24.75	230.00	-17.50
Min, Max	165.0, 348.0	-105.0, 61.5	178.0, 342.0	-52.5, 77.0	165.0, 348.0	-105.0, 77.0
Week 104						
n	1	1	2	2	3	3
Mean (SD)	193.00 (NA)	-44.50 (NA)	256.00 (49.497)	-21.25 (78.135)	235.00 (50.478)	-29.00 (56.857)
Median	193.00	-44.50	256.00	-21.25	221.00	-44.50
Min, Max	193.0, 193.0	-44.5, -44.5	221.0, 291.0	-76.5, 34.0	193.0, 291.0	-76.5, 34.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2x$ ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:17

Data cutoff date of 31Mar2023

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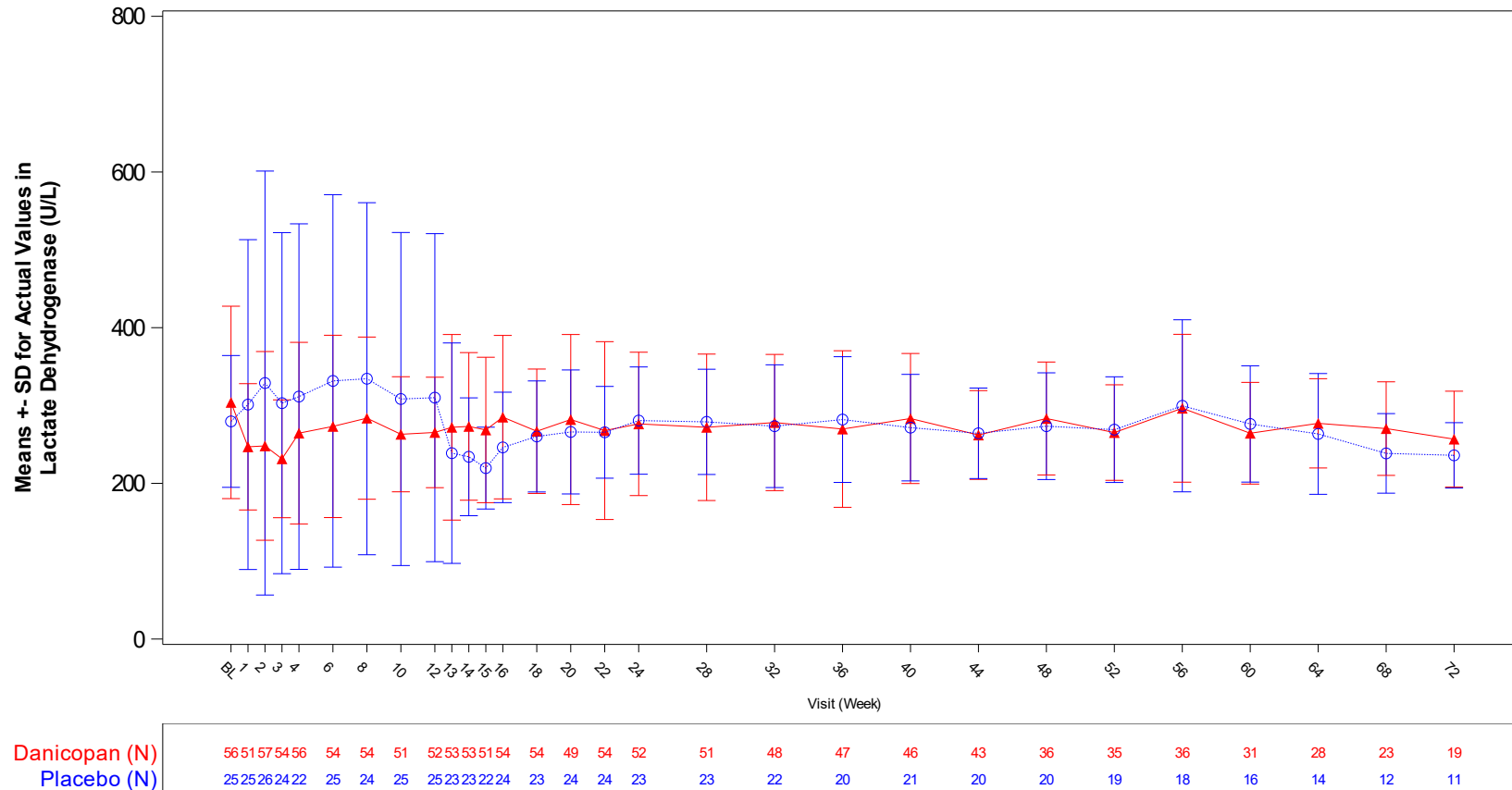
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Post-hoc Analysen Studie ALPHA

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ALXN2040-PNH-301

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Figure GERMANY.2.2.4.3
Plots of Mean \pm SD for Actual Values in Lactate Dehydrogenase (LDH) During 72 Weeks
Modified Randomized Set



Baseline is defined as the average of all available assessments prior to the first dose of study drug.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium \geq 6 mmol/L and LDH \geq 2x ULN are defined as having TTH.

Source: ADaM.ADLB

Run Date: 2024-04-26T15:31:16

Data cutoff date of 31Mar2023

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Table GERMANY.2.3.3.1
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Visit	Statistics	Danicopan (N = 56)	Placebo (N = 25)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-54.87 (6.778)	-26.25 (9.681)	-28.61 (11.694)	0.0168
	95% CI for LS MEAN	(-68.36, -41.38)	(-45.55, -6.96)	(-51.92, -5.31)	
Week 2	LS Mean (SEM)	-62.95 (15.827)	6.26 (23.626)	-69.20 (28.387)	0.0170
	95% CI for LS MEAN	(-94.45, -31.45)	(-40.77, 53.28)	(-125.71, -12.70)	
Week 3	LS Mean (SEM)	-70.27 (7.931)	-26.41 (11.841)	-43.86 (14.153)	0.0027
	95% CI for LS MEAN	(-86.07, -54.47)	(-50.00, -2.82)	(-72.06, -15.66)	
Week 4	LS Mean (SEM)	-44.14 (8.114)	-6.77 (12.841)	-37.37 (15.116)	0.0222
	95% CI for LS MEAN	(-61.12, -27.16)	(-33.41, 19.88)	(-68.84, -5.91)	
Week 6	LS Mean (SEM)	-30.91 (13.067)	5.36 (19.467)	-36.26 (23.383)	0.1251
	95% CI for LS MEAN	(-56.93, -4.89)	(-33.41, 44.13)	(-82.84, 10.31)	
Week 8	LS Mean (SEM)	-16.91 (11.211)	8.83 (16.858)	-25.74 (20.175)	0.2060
	95% CI for LS MEAN	(-39.26, 5.43)	(-24.76, 42.42)	(-65.95, 14.47)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. An Unstructured covariance structure was used.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM.

Source: ADaM.ADSL, ADaM.ADEFF

Run Date: 2024-04-26T15:50:06

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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Table GERMANY.2.3.3.1
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Visit	Statistics	Danicopan (N = 56)	Placebo (N = 25)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-26.59 (7.759)	-15.34 (11.443)	-11.25 (13.714)	0.4147
	95% CI for LS MEAN	(-42.04, -11.13)	(-38.15, 7.47)	(-38.59, 16.09)	
Week 12	LS Mean (SEM)	-25.04 (7.887)	-15.21 (11.604)	-9.82 (13.926)	0.4835
	95% CI for LS MEAN	(-40.82, -9.25)	(-38.46, 8.04)	(-37.72, 18.08)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. An Unstructured covariance structure was used.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM.

Source: ADaM.ADSL, ADaM.ADEFF

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Data cutoff date of 31Mar2023

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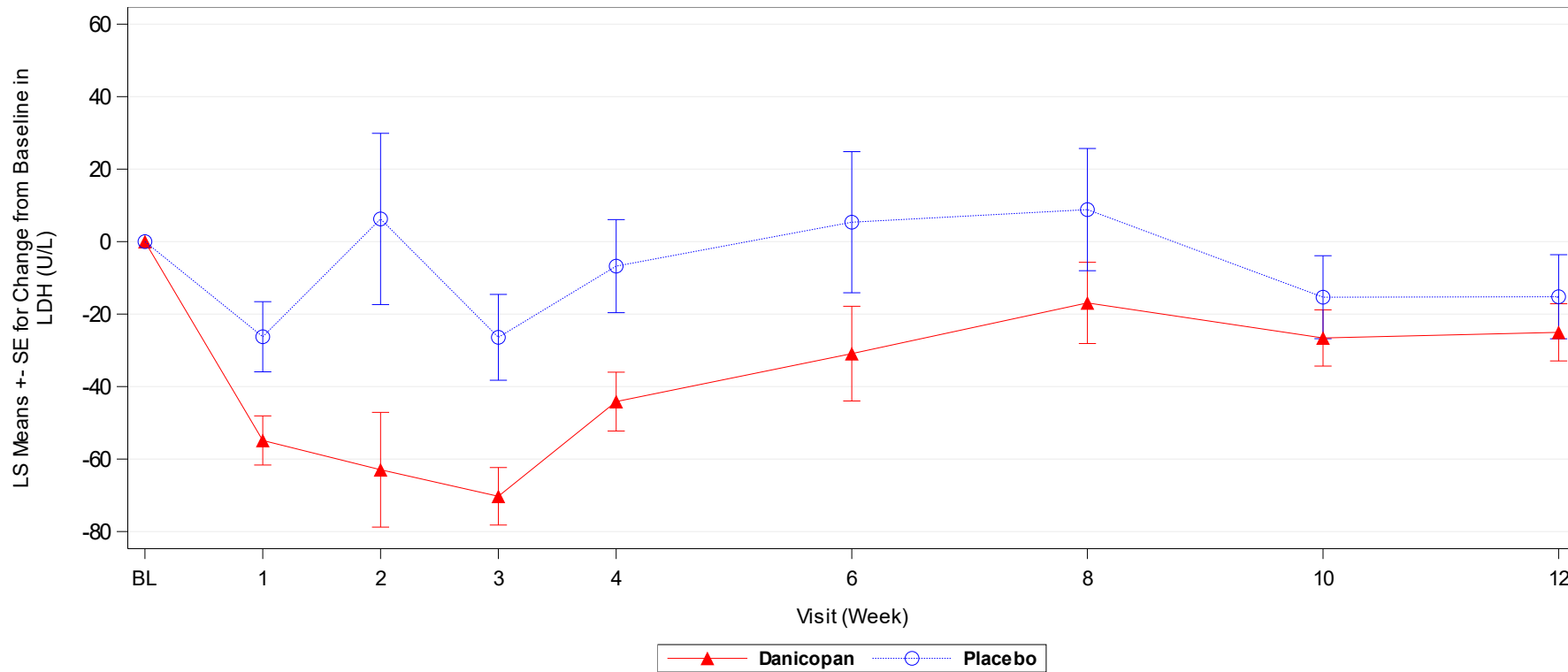
Post-hoc Analysen Studie ALPHA

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ALXN2040-PNH-301

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Figure GERMANY.2.2.4.1

Plots of LS Means \pm SE for Change from Baseline in Lactate Dehydrogenase (LDH) During 12-Week of Treatment Period 1
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium \geq 6 mmol/L and LDH \geq 2x ULN are defined as having TTH.

Source: ADaM.ADSL, ADaM.ADEFF

Run Date: 2024-04-26T15:31:25

Data cutoff date of 31Mar2023

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.3 Veränderung des LDH-Wertes

1.3.1 Subgruppenanalysen – Veränderung des LDH-Wertes gegenüber Baseline

Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.2
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	369.45 (159.887)		257.72 (49.627)		337.02 (145.635)	
Median	321.00		247.00		287.50	
Min, Max	200.0, 809.0		186.5, 356.5		186.5, 809.0	
Week 1						
n	22	21	9	9		
Mean (SD)	285.86 (94.199)	-97.21 (98.130)	254.11 (58.591)	-3.61 (64.316)		
Median	254.00	-76.50	241.00	-13.00		
Min, Max	161.0, 532.0	-295.5, 87.0	198.0, 395.0	-72.5, 155.0		
Week 2						
n	23	22	9	9		
Mean (SD)	293.13 (167.961)	-106.32 (103.097)	347.22 (319.893)	89.50 (325.473)		
Median	245.00	-71.75	235.00	-11.00		
Min, Max	126.0, 953.0	-307.5, 23.0	192.0, 1195.0	-67.5, 955.0		

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.2
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	23	22	9	9		
Mean (SD)	257.61 (86.881)	-115.68 (113.394)	255.89 (71.785)	-1.83 (78.499)		
Median	236.00	-90.50	240.00	-10.00		
Min, Max	121.0, 540.0	-398.5, 24.0	187.0, 433.0	-77.5, 193.0		
Week 4						
n	22	21	8	8		
Mean (SD)	320.41 (151.710)	-75.74 (105.750)	248.13 (27.777)	-11.81 (34.730)		
Median	281.00	-38.00	254.00	1.00		
Min, Max	108.0, 847.0	-259.5, 114.0	200.0, 276.0	-82.5, 17.5		
Week 6						
n	23	22	9	9		
Mean (SD)	319.30 (154.145)	-59.68 (93.539)	300.89 (204.716)	43.17 (212.999)		
Median	275.00	-55.25	240.00	-9.50		
Min, Max	124.0, 794.0	-238.5, 142.0	188.0, 843.0	-98.5, 603.0		

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Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	21	20	9	9		
Mean (SD)	327.81 (89.963)	-50.90 (104.686)	306.67 (145.896)	48.94 (153.950)		
Median	351.00	-26.75	259.00	17.50		
Min, Max	145.0, 543.0	-337.5, 74.0	204.0, 672.0	-113.5, 432.0		
Week 10						
n	20	19	9	9		
Mean (SD)	295.00 (84.520)	-59.45 (91.190)	253.78 (65.979)	-3.94 (79.701)		
Median	272.00	-23.50	236.00	-17.50		
Min, Max	206.0, 589.0	-220.0, 45.0	200.0, 422.0	-98.5, 182.0		
Week 12						
n	23	22	9	9		
Mean (SD)	297.96 (74.613)	-69.86 (122.067)	246.22 (31.937)	-11.50 (45.418)		
Median	282.00	-18.50	250.00	-7.50		
Min, Max	171.0, 519.0	-379.5, 67.0	188.0, 304.0	-84.5, 64.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	22	21	8	8		
Mean (SD)	309.50 (161.985)	-60.93 (203.600)	227.25 (46.833)	-18.13 (42.566)		
Median	255.50	-43.00	220.50	-4.25		
Min, Max	129.0, 882.0	-496.5, 627.5	162.0, 315.0	-103.0, 27.5		
Week 14						
n	23	22	9	9		
Mean (SD)	319.22 (110.199)	-47.91 (154.355)	230.44 (42.878)	-27.28 (54.636)		
Median	276.00	-20.25	224.00	-1.50		
Min, Max	153.0, 587.0	-442.5, 208.5	158.0, 310.0	-135.5, 22.5		
Week 15						
n	22	21	9	9		
Mean (SD)	299.09 (100.015)	-78.14 (131.818)	223.67 (29.933)	-34.06 (50.945)		
Median	277.50	-55.00	221.00	-19.50		
Min, Max	155.0, 627.0	-486.5, 165.5	167.0, 276.0	-122.5, 29.0		

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Table GERMANY.2.6.8.2

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	22	21	9	9		
Mean (SD)	318.18 (105.397)	-52.52 (127.091)	241.78 (34.546)	-15.94 (58.661)		
Median	298.50	-39.50	254.00	10.50		
Min, Max	177.0, 582.0	-258.5, 243.5	186.0, 297.0	-108.0, 50.0		
Week 18						
n	23	22	8	8		
Mean (SD)	306.00 (83.174)	-62.68 (114.137)	267.25 (34.858)	13.25 (62.454)		
Median	288.00	-24.00	282.50	38.25		
Min, Max	172.0, 568.0	-297.5, 113.5	200.0, 301.0	-94.0, 67.5		
Week 20						
n	20	19	9	9		
Mean (SD)	327.05 (145.643)	-61.42 (109.702)	261.33 (51.549)	3.61 (62.872)		
Median	280.50	-52.00	269.00	7.50		
Min, Max	168.0, 725.0	-243.5, 235.5	185.0, 318.0	-109.0, 84.5		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	23	22	9	9		
Mean (SD)	310.00 (151.089)	-61.64 (106.150)	278.44 (37.892)	20.72 (62.758)		
Median	283.00	-39.25	289.00	49.00		
Min, Max	147.0, 906.0	-307.0, 171.5	199.0, 314.0	-95.0, 83.5		
Week 24						
n	21	20	9	9		
Mean (SD)	306.33 (107.282)	-77.08 (139.260)	289.44 (61.934)	31.72 (70.071)		
Median	277.00	-30.00	298.00	51.00		
Min, Max	170.0, 573.0	-491.5, 93.5	183.0, 366.0	-88.0, 135.5		
Week 28						
n	22	22	9	9	31	31
Mean (SD)	311.27 (118.642)	-58.18 (150.303)	289.78 (53.513)	32.06 (81.262)	305.03 (103.514)	-31.98 (138.954)
Median	271.00	-44.00	304.00	27.50	286.00	-13.00
Min, Max	174.0, 632.0	-467.5, 370.5	193.0, 342.0	-101.0, 143.5	174.0, 632.0	-467.5, 370.5

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Post-hoc Analysen Studie ALPHA

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ALXN2040-PNH-301

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Modified Randomized Set

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	21	20	8	8	29	28
Mean (SD)	318.24 (100.886)	-66.83 (119.475)	278.88 (90.506)	17.75 (92.126)	307.38 (98.175)	-42.66 (117.299)
Median	305.00	-26.75	271.00	21.25	279.00	-7.50
Min, Max	161.0, 512.0	-331.5, 79.5	154.0, 421.0	-103.0, 141.0	154.0, 512.0	-331.5, 141.0
Week 36						
n	20	19	8	8	28	27
Mean (SD)	283.55 (62.406)	-51.18 (85.550)	261.50 (60.837)	0.38 (57.241)	277.25 (61.669)	-35.91 (80.775)
Median	268.00	-59.50	257.50	-7.75	268.00	-20.50
Min, Max	176.0, 404.0	-229.5, 62.0	166.0, 354.0	-62.5, 107.0	166.0, 404.0	-229.5, 107.0
Week 40						
n	21	20	7	7	28	27
Mean (SD)	312.00 (90.853)	-43.50 (131.827)	269.29 (71.074)	6.14 (73.905)	301.32 (87.130)	-30.63 (120.214)
Median	293.00	-30.75	267.00	-7.50	292.50	-20.50
Min, Max	170.0, 623.0	-395.5, 147.0	177.0, 360.0	-117.0, 120.0	170.0, 623.0	-395.5, 147.0

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Modified Randomized Set

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	20	19	7	7	27	26
Mean (SD)	284.85 (49.924)	-75.76 (113.504)	275.14 (51.454)	3.36 (71.394)	282.33 (49.509)	-54.46 (108.536)
Median	290.00	-46.50	294.00	21.00	291.00	-35.75
Min, Max	181.0, 394.0	-443.5, 78.0	176.0, 328.0	-118.0, 81.0	176.0, 394.0	-443.5, 81.0
Week 48						
n	17	17	7	7	24	24
Mean (SD)	318.53 (76.051)	-14.26 (108.401)	297.86 (73.969)	26.07 (71.074)	312.50 (74.451)	-2.50 (99.211)
Median	308.00	-17.50	288.00	36.00	305.00	0.00
Min, Max	180.0, 455.0	-198.5, 230.0	193.0, 413.0	-101.0, 125.5	180.0, 455.0	-198.5, 230.0
Week 52						
n	17	17	6	6	23	23
Mean (SD)	292.35 (53.197)	-64.71 (120.014)	285.83 (45.181)	11.83 (74.988)	290.65 (50.305)	-44.74 (113.728)
Median	299.00	-39.50	280.00	37.25	298.00	-36.00
Min, Max	201.0, 387.0	-436.5, 67.0	227.0, 347.0	-92.5, 100.0	201.0, 387.0	-436.5, 100.0

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Modified Randomized Set

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	17	17	5	5	22	22
Mean (SD)	321.18 (67.918)	-35.88 (136.761)	307.40 (55.257)	28.00 (83.668)	318.05 (64.274)	-21.36 (127.806)
Median	310.00	-41.50	316.00	26.00	313.00	-11.75
Min, Max	199.0, 439.0	-329.5, 225.0	245.0, 389.0	-87.5, 149.0	199.0, 439.0	-329.5, 225.0
Week 60						
n	16	16	4	4	20	20
Mean (SD)	280.75 (56.134)	-52.72 (81.733)	257.50 (64.029)	-31.75 (74.210)	276.10 (56.798)	-48.53 (78.851)
Median	281.50	-45.25	266.00	-21.75	281.50	-45.25
Min, Max	168.0, 391.0	-199.5, 71.0	175.0, 323.0	-119.0, 35.5	168.0, 391.0	-199.5, 71.0
Week 64						
n	14	14	3	3	17	17
Mean (SD)	308.14 (43.638)	-39.71 (88.533)	266.67 (76.107)	-0.17 (87.625)	300.82 (50.368)	-32.74 (87.004)
Median	323.50	-25.00	262.00	43.00	319.00	-24.50
Min, Max	230.0, 366.0	-227.5, 68.0	193.0, 345.0	-101.0, 57.5	193.0, 366.0	-227.5, 68.0

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.2
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	10	10	3	3	13	13
Mean (SD)	299.70 (44.982)	-39.15 (89.282)	249.33 (86.662)	-17.50 (92.848)	288.08 (57.071)	-34.15 (86.634)
Median	296.50	-27.50	233.00	14.00	293.00	-14.00
Min, Max	250.0, 412.0	-192.0, 93.0	172.0, 343.0	-122.0, 55.5	172.0, 412.0	-192.0, 93.0
Week 72						
n	8	8	2	2	10	10
Mean (SD)	278.13 (37.802)	-58.00 (109.083)	207.50 (64.347)	-49.00 (117.380)	264.00 (49.580)	-56.20 (103.924)
Median	278.00	-22.25	207.50	-49.00	270.50	-22.25
Min, Max	222.0, 340.0	-206.5, 69.5	162.0, 253.0	-132.0, 34.0	162.0, 340.0	-206.5, 69.5
Week 80						
n	8	8	1	1	9	9
Mean (SD)	295.38 (47.313)	-40.75 (96.756)	244.00 (NA)	25.00 (NA)	289.67 (47.455)	-33.44 (93.122)
Median	306.50	-48.75	244.00	25.00	296.00	-37.50
Min, Max	217.0, 355.0	-162.5, 120.0	244.0, 244.0	25.0, 25.0	217.0, 355.0	-162.5, 120.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.2
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	5	5	1	1	6	6
Mean (SD)	365.40 (96.824)	71.00 (155.303)	263.00 (NA)	44.00 (NA)	348.33 (96.164)	66.50 (139.343)
Median	350.00	39.50	263.00	44.00	322.00	41.75
Min, Max	292.0, 529.0	-91.0, 329.0	263.0, 263.0	44.0, 44.0	263.0, 529.0	-91.0, 329.0
Week 96						
n	2	2	1	1	3	3
Mean (SD)	288.00 (84.853)	-85.50 (27.577)	296.00 (NA)	77.00 (NA)	290.67 (60.178)	-31.33 (95.824)
Median	288.00	-85.50	296.00	77.00	296.00	-66.00
Min, Max	228.0, 348.0	-105.0, -66.0	296.0, 296.0	77.0, 77.0	228.0, 348.0	-105.0, 77.0

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Alexion Pharmaceuticals, Inc.
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Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		16		50	
Mean (SD)	261.64 (66.998)		291.84 (98.532)		271.31 (78.725)	
Median	244.00		286.75		247.25	
Min, Max	140.0, 408.0		139.0, 506.0		139.0, 506.0	
Week 1						
n	29	29	16	15		
Mean (SD)	217.41 (54.818)	-42.93 (33.200)	327.69 (260.617)	-30.07 (93.736)		
Median	206.00	-35.00	253.50	-11.00		
Min, Max	137.0, 348.0	-120.5, 9.5	128.0, 1260.0	-332.0, 64.0		
Week 2						
n	34	34	17	16		
Mean (SD)	217.65 (61.360)	-44.00 (45.631)	319.18 (253.842)	-31.47 (97.708)		
Median	201.50	-42.75	262.00	-5.25		
Min, Max	117.0, 361.0	-133.5, 66.5	144.0, 1260.0	-345.0, 76.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	31	31	15	14		
Mean (SD)	212.19 (60.660)	-48.92 (36.165)	331.33 (271.333)	-15.57 (80.661)		
Median	201.00	-49.00	271.00	-9.75		
Min, Max	122.0, 326.0	-142.5, 9.5	123.0, 1260.0	-185.0, 176.0		
Week 4						
n	34	34	14	13		
Mean (SD)	228.35 (67.812)	-33.29 (51.804)	347.64 (274.405)	11.15 (33.117)		
Median	211.50	-27.00	306.50	14.00		
Min, Max	138.0, 389.0	-161.5, 98.5	123.0, 1260.0	-29.0, 76.5		
Week 6						
n	31	31	16	15		
Mean (SD)	238.90 (62.936)	-22.09 (49.745)	348.88 (261.450)	10.57 (77.629)		
Median	243.00	-25.50	273.00	22.50		
Min, Max	140.0, 405.0	-148.0, 78.0	130.0, 1260.0	-167.0, 125.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	33	33	15	14		
Mean (SD)	255.76 (104.086)	-6.62 (69.597)	351.07 (266.581)	15.32 (41.730)		
Median	227.00	-19.50	282.00	7.25		
Min, Max	146.0, 629.0	-110.0, 255.5	122.0, 1260.0	-43.0, 113.5		
Week 10						
n	31	31	16	15		
Mean (SD)	242.45 (58.882)	-12.11 (49.776)	339.06 (260.974)	0.10 (73.354)		
Median	227.00	-12.00	262.50	8.50		
Min, Max	148.0, 399.0	-177.0, 82.5	155.0, 1260.0	-137.0, 191.5		
Week 12						
n	29	29	16	15		
Mean (SD)	239.59 (56.898)	-8.84 (51.605)	346.00 (258.174)	7.50 (34.250)		
Median	243.00	-13.00	309.00	10.00		
Min, Max	135.0, 342.0	-157.0, 99.5	128.0, 1260.0	-45.0, 80.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	31	31	15	14		
Mean (SD)	245.42 (67.126)	-9.90 (54.158)	244.93 (174.063)	-69.75 (63.664)		
Median	247.00	-5.00	219.00	-53.25		
Min, Max	136.0, 407.0	-186.0, 133.5	122.0, 854.0	-196.0, 3.5		
Week 14						
n	30	30	14	13		
Mean (SD)	237.83 (62.738)	-14.23 (66.566)	236.50 (92.458)	-50.19 (64.424)		
Median	235.50	-16.25	216.00	-21.00		
Min, Max	135.0, 452.0	-187.0, 214.5	118.0, 501.0	-189.0, 16.0		
Week 15						
n	29	29	13	12		
Mean (SD)	245.48 (82.431)	-5.17 (84.392)	216.77 (64.954)	-58.33 (59.759)		
Median	236.00	-13.00	210.00	-34.00		
Min, Max	134.0, 521.0	-198.0, 283.5	123.0, 361.0	-168.0, 11.5		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	32	32	15	14		
Mean (SD)	262.28 (100.024)	8.26 (83.171)	248.80 (86.971)	-27.32 (98.841)		
Median	233.50	-9.00	239.00	-18.00		
Min, Max	144.0, 639.0	-93.0, 332.0	121.0, 439.0	-182.0, 252.0		
Week 18						
n	31	31	15	14		
Mean (SD)	237.94 (64.563)	-12.93 (55.952)	256.53 (85.708)	-18.39 (52.215)		
Median	224.00	-2.50	226.00	-19.00		
Min, Max	159.0, 419.0	-188.0, 100.5	141.0, 461.0	-111.0, 93.5		
Week 20						
n	29	29	15	14		
Mean (SD)	250.93 (60.077)	-6.32 (50.911)	268.87 (94.262)	-7.96 (81.480)		
Median	257.00	9.00	250.00	-5.50		
Min, Max	157.0, 400.0	-142.0, 89.5	137.0, 535.0	-127.0, 229.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
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Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	31	31	15	14		
Mean (SD)	236.48 (62.773)	-20.19 (52.722)	257.87 (68.707)	-18.61 (80.306)		
Median	241.00	-13.00	245.00	0.25		
Min, Max	108.0, 405.0	-172.0, 78.5	169.0, 416.0	-180.0, 110.0		
Week 24						
n	31	31	14	13		
Mean (SD)	256.10 (75.426)	-0.58 (64.860)	275.00 (74.744)	-12.04 (85.529)		
Median	260.00	-3.33	249.00	6.50		
Min, Max	130.0, 468.0	-161.0, 142.0	188.0, 442.0	-125.0, 136.0		
Week 28						
n	29	29	14	13	43	42
Mean (SD)	242.21 (55.795)	-8.80 (64.227)	272.07 (76.409)	-14.12 (83.483)	251.93 (63.899)	-10.45 (69.736)
Median	238.00	-10.00	252.50	1.00	242.00	-8.75
Min, Max	134.0, 339.0	-176.0, 114.0	173.0, 433.0	-161.0, 127.0	134.0, 433.0	-176.0, 127.0

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Modified Randomized Set

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	27	27	14	13	41	40
Mean (SD)	247.00 (60.854)	-7.39 (53.772)	270.36 (74.851)	-17.73 (96.545)	254.98 (65.982)	-10.75 (69.424)
Median	238.00	-6.00	246.50	-1.00	243.00	-4.00
Min, Max	150.0, 366.0	-185.0, 101.0	192.0, 421.0	-167.0, 153.5	150.0, 421.0	-185.0, 153.5
Week 36						
n	27	27	12	11	39	38
Mean (SD)	259.59 (121.642)	4.71 (111.122)	295.58 (91.689)	23.91 (67.196)	270.67 (113.318)	10.27 (99.876)
Median	244.00	-12.00	266.50	9.50	245.00	-2.25
Min, Max	131.0, 804.0	-164.0, 470.0	172.0, 478.0	-52.0, 172.0	131.0, 804.0	-164.0, 470.0
Week 40						
n	25	25	14	13	39	38
Mean (SD)	259.20 (69.886)	2.93 (71.155)	272.71 (69.780)	-15.12 (77.668)	264.05 (69.236)	-3.25 (72.910)
Median	245.00	-0.50	246.00	-18.00	245.00	-7.00
Min, Max	170.0, 516.0	-193.0, 209.0	170.0, 394.0	-164.0, 94.5	170.0, 516.0	-193.0, 209.0

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.2
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	23	23	13	12	36	35
Mean (SD)	242.35 (56.381)	-8.14 (39.323)	258.46 (62.639)	-21.13 (78.490)	248.17 (58.352)	-12.60 (55.071)
Median	243.00	-14.00	247.00	-1.75	245.00	-14.00
Min, Max	149.0, 329.0	-54.5, 93.0	164.0, 373.0	-180.0, 68.0	149.0, 373.0	-180.0, 93.0
Week 48						
n	19	19	13	12	32	31
Mean (SD)	251.84 (53.617)	-1.18 (41.918)	260.31 (64.570)	-24.54 (65.277)	255.28 (57.454)	-10.23 (52.444)
Median	250.00	-8.50	249.00	-16.75	249.50	-8.50
Min, Max	157.0, 346.0	-76.0, 87.0	170.0, 355.0	-164.0, 62.0	157.0, 355.0	-164.0, 87.0
Week 52						
n	18	18	13	12	31	30
Mean (SD)	239.67 (58.644)	-8.64 (34.019)	261.23 (76.508)	-24.63 (92.016)	248.71 (66.387)	-15.03 (62.876)
Median	217.00	-13.25	242.00	-15.50	241.00	-13.25
Min, Max	155.0, 356.0	-57.5, 82.5	175.0, 455.0	-189.0, 149.0	155.0, 455.0	-189.0, 149.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:18

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.2
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	19	19	13	12	32	31
Mean (SD)	274.42 (111.144)	21.39 (85.763)	296.62 (127.480)	12.63 (125.481)	283.44 (116.560)	18.00 (101.021)
Median	251.00	-3.50	255.00	-2.75	251.00	-3.50
Min, Max	153.0, 635.0	-42.0, 339.0	186.0, 673.0	-149.0, 339.0	153.0, 673.0	-149.0, 339.0
Week 60						
n	15	15	12	11	27	26
Mean (SD)	246.93 (71.635)	4.57 (70.109)	282.58 (79.506)	5.77 (73.613)	262.78 (75.917)	5.08 (70.146)
Median	237.00	-4.50	247.50	6.50	242.00	-3.00
Min, Max	161.0, 444.0	-74.0, 204.0	166.0, 444.0	-151.0, 110.0	161.0, 444.0	-151.0, 204.0
Week 64						
n	14	14	11	10	25	24
Mean (SD)	246.07 (53.434)	5.89 (49.095)	262.64 (81.623)	-14.65 (62.110)	253.36 (66.279)	-2.67 (54.579)
Median	257.50	-10.50	231.00	-16.75	236.00	-14.25
Min, Max	151.0, 319.0	-64.0, 84.0	167.0, 421.0	-131.5, 87.0	151.0, 421.0	-131.5, 87.0

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Source: ADaM.ADSL, ADaM.ADLB
Run Date: 2024-04-26T15:50:18
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Data cutoff date of 31Mar2023
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.2
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	13	13	9	9	22	22
Mean (SD)	247.77 (61.936)	14.81 (50.831)	234.89 (40.714)	-21.94 (74.070)	242.50 (53.530)	-0.23 (62.518)
Median	275.00	26.50	236.00	-12.00	263.00	21.75
Min, Max	137.0, 333.0	-73.0, 99.5	162.0, 285.0	-131.5, 85.0	137.0, 333.0	-131.5, 99.5
Week 72						
n	11	11	9	9	20	20
Mean (SD)	241.27 (72.132)	10.95 (41.124)	242.33 (37.911)	-14.50 (74.925)	241.75 (57.826)	-0.50 (58.503)
Median	228.00	16.00	245.00	-16.00	239.50	-2.50
Min, Max	142.0, 403.0	-57.0, 78.0	182.0, 307.0	-138.0, 120.0	142.0, 403.0	-138.0, 120.0
Week 80						
n	9	9	7	7	16	16
Mean (SD)	260.78 (86.036)	30.72 (77.854)	243.00 (78.083)	-18.57 (39.485)	253.00 (80.434)	9.16 (67.038)
Median	272.00	11.50	239.00	-15.00	240.50	-9.25
Min, Max	153.0, 449.0	-51.0, 215.5	171.0, 389.0	-72.5, 52.0	153.0, 449.0	-72.5, 215.5

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.2
Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	6	6	12	12
Mean (SD)	255.67 (56.916)	25.75 (47.621)	238.83 (29.020)	-1.08 (52.165)	247.25 (43.961)	12.33 (49.639)
Median	246.00	10.25	232.50	6.25	244.00	9.50
Min, Max	176.0, 350.0	-18.5, 116.5	211.0, 274.0	-93.5, 59.0	176.0, 350.0	-93.5, 116.5
Week 96						
n	5	5	5	5	10	10
Mean (SD)	235.20 (47.620)	7.80 (31.348)	232.00 (66.042)	-19.90 (37.053)	233.60 (54.306)	-6.05 (35.498)
Median	246.00	-0.50	227.00	-25.50	228.50	-14.00
Min, Max	165.0, 295.0	-17.5, 61.5	178.0, 342.0	-52.5, 43.0	165.0, 342.0	-52.5, 61.5
Week 104						
n	1	1	2	2	3	3
Mean (SD)	193.00 (NA)	-44.50 (NA)	256.00 (49.497)	-21.25 (78.135)	235.00 (50.478)	-29.00 (56.857)
Median	193.00	-44.50	256.00	-21.25	221.00	-44.50
Min, Max	193.0, 193.0	-44.5, -44.5	221.0, 291.0	-76.5, 34.0	193.0, 291.0	-76.5, 34.0

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Source: ADaM.ADSL, ADaM.ADLB
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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Table GERMANY.2.6.8.3

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	40		20		60	
Mean (SD)	311.56 (142.373)		289.20 (90.763)		304.11 (127.141)	
Median	259.00		277.50		261.00	
Min, Max	140.0, 809.0		139.0, 506.0		139.0, 809.0	
Week 1						
n	36	35	20	20		
Mean (SD)	252.53 (91.192)	-70.44 (85.104)	267.70 (77.195)	-21.50 (91.278)		
Median	231.00	-42.00	248.50	-12.00		
Min, Max	137.0, 532.0	-295.5, 87.0	128.0, 404.0	-332.0, 155.0		
Week 2						
n	41	40	20	20		
Mean (SD)	251.07 (137.006)	-78.03 (88.558)	305.75 (221.032)	16.55 (237.578)		
Median	224.00	-57.75	261.50	-9.00		
Min, Max	117.0, 953.0	-307.5, 66.5	144.0, 1195.0	-345.0, 955.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:21

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.3

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	38	37	19	19		
Mean (SD)	226.71 (79.639)	-91.57 (93.682)	268.47 (88.147)	-9.32 (85.970)		
Median	214.50	-63.00	264.00	-10.00		
Min, Max	122.0, 540.0	-398.5, 9.0	123.0, 433.0	-185.0, 193.0		
Week 4						
n	40	39	16	16		
Mean (SD)	264.05 (128.781)	-61.84 (85.603)	270.19 (75.722)	-0.84 (35.219)		
Median	231.50	-40.50	270.00	1.00		
Min, Max	138.0, 847.0	-259.5, 114.0	123.0, 389.0	-82.5, 76.5		
Week 6						
n	38	37	19	19		
Mean (SD)	276.05 (131.482)	-45.85 (81.865)	308.00 (158.295)	30.21 (157.189)		
Median	248.50	-28.00	264.00	1.00		
Min, Max	140.0, 794.0	-238.5, 142.0	130.0, 843.0	-167.0, 603.0		

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.3

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	38	37	18	18		
Mean (SD)	282.45 (107.462)	-34.21 (93.442)	308.11 (124.174)	35.56 (111.421)		
Median	245.50	-32.50	278.50	15.50		
Min, Max	146.0, 629.0	-337.5, 255.5	122.0, 672.0	-113.5, 432.0		
Week 10						
n	36	35	19	19		
Mean (SD)	254.86 (77.003)	-45.08 (76.462)	280.84 (88.254)	3.05 (82.498)		
Median	237.50	-20.50	259.00	9.00		
Min, Max	148.0, 589.0	-220.0, 55.0	155.0, 459.0	-137.0, 191.5		
Week 12						
n	38	37	19	19		
Mean (SD)	259.79 (73.829)	-51.52 (100.619)	282.21 (78.320)	4.42 (42.108)		
Median	250.50	-22.00	272.00	10.00		
Min, Max	135.0, 519.0	-379.5, 70.5	128.0, 410.0	-84.5, 80.0		

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Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.8.3

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	39	38	17	17		
Mean (SD)	258.36 (87.393)	-53.63 (111.451)	213.00 (51.004)	-54.91 (66.370)		
Median	240.00	-14.25	219.00	-31.00		
Min, Max	136.0, 639.0	-496.5, 133.5	122.0, 315.0	-196.0, 27.5		
Week 14						
n	39	38	17	17		
Mean (SD)	268.69 (95.038)	-43.76 (116.696)	225.41 (53.053)	-43.82 (65.505)		
Median	249.00	-17.25	220.00	-15.00		
Min, Max	135.0, 587.0	-442.5, 214.5	118.0, 322.0	-189.0, 22.5		
Week 15						
n	36	35	16	16		
Mean (SD)	268.11 (100.463)	-47.71 (126.528)	210.94 (46.349)	-56.03 (58.603)		
Median	244.50	-26.00	216.00	-34.00		
Min, Max	134.0, 627.0	-486.5, 283.5	123.0, 279.0	-168.0, 29.0		

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Post-hoc Analysen Studie ALPHA

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Table GERMANY.2.6.8.3

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< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	39	38	18	18		
Mean (SD)	271.77 (92.346)	-38.55 (94.663)	248.44 (78.149)	-24.39 (92.039)		
Median	247.00	-20.75	243.00	-18.00		
Min, Max	144.0, 582.0	-258.5, 210.5	121.0, 439.0	-182.0, 252.0		
Week 18						
n	40	39	17	17		
Mean (SD)	266.48 (84.953)	-44.07 (94.366)	259.88 (80.781)	-12.09 (55.732)		
Median	258.50	-8.00	236.00	-18.00		
Min, Max	159.0, 568.0	-297.5, 88.0	141.0, 461.0	-111.0, 93.5		
Week 20						
n	36	35	18	18		
Mean (SD)	285.25 (122.671)	-35.40 (92.195)	265.67 (84.998)	-7.17 (75.134)		
Median	260.50	-26.00	253.00	-5.50		
Min, Max	157.0, 725.0	-243.5, 235.5	137.0, 535.0	-127.0, 229.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.
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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.3

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	39	38	18	18		
Mean (SD)	270.92 (127.722)	-44.97 (90.008)	263.00 (62.084)	-9.83 (76.605)		
Median	254.00	-23.75	260.00	7.50		
Min, Max	108.0, 906.0	-307.0, 171.5	169.0, 416.0	-180.0, 110.0		
Week 24						
n	37	36	17	17		
Mean (SD)	270.86 (99.226)	-48.37 (116.704)	281.29 (67.888)	0.59 (81.833)		
Median	262.00	-23.75	264.00	45.00		
Min, Max	130.0, 573.0	-491.5, 130.5	188.0, 442.0	-125.0, 136.0		
Week 28						
n	37	37	17	17	54	54
Mean (SD)	279.81 (104.602)	-33.47 (128.086)	277.12 (70.637)	-3.59 (80.568)	278.96 (94.551)	-24.06 (115.323)
Median	257.00	-18.00	274.00	13.00	262.00	-7.50
Min, Max	134.0, 632.0	-467.5, 370.5	173.0, 433.0	-161.0, 127.0	134.0, 632.0	-467.5, 370.5

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Source: ADaM.ADSL, ADaM.ADLB
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.3

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	34	33	17	17	51	50
Mean (SD)	278.18 (96.111)	-46.44 (103.646)	280.88 (79.510)	0.18 (101.622)	279.08 (90.118)	-30.59 (104.332)
Median	247.00	-15.50	250.00	9.00	249.00	-9.25
Min, Max	150.0, 512.0	-331.5, 101.0	192.0, 421.0	-167.0, 153.5	150.0, 512.0	-331.5, 153.5
Week 36						
n	32	31	15	15	47	46
Mean (SD)	255.44 (61.946)	-38.17 (79.245)	289.33 (85.918)	17.97 (66.369)	266.26 (71.329)	-19.87 (79.151)
Median	250.00	-28.50	277.00	-2.00	252.00	-13.50
Min, Max	131.0, 404.0	-229.5, 111.7	172.0, 478.0	-62.5, 172.0	131.0, 478.0	-229.5, 172.0
Week 40						
n	33	32	16	16	49	48
Mean (SD)	278.39 (84.149)	-32.18 (112.966)	272.38 (65.576)	-10.44 (73.975)	276.43 (77.927)	-24.93 (101.345)
Median	257.00	-9.00	258.50	-19.25	257.00	-12.75
Min, Max	170.0, 623.0	-395.5, 153.0	170.0, 394.0	-164.0, 120.0	170.0, 623.0	-395.5, 153.0

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Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.3

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	30	29	16	16	46	45
Mean (SD)	256.23 (56.426)	-55.13 (97.469)	263.19 (57.940)	-11.81 (75.000)	258.65 (56.410)	-39.73 (91.668)
Median	265.00	-31.00	249.50	5.75	260.50	-25.00
Min, Max	149.0, 394.0	-443.5, 93.0	164.0, 373.0	-180.0, 81.0	149.0, 394.0	-443.5, 93.0
Week 48						
n	25	25	16	16	41	41
Mean (SD)	279.16 (77.268)	-16.72 (87.556)	271.44 (68.979)	-5.94 (69.519)	276.15 (73.355)	-12.51 (80.251)
Median	271.00	-12.00	270.50	0.00	271.00	-9.50
Min, Max	157.0, 455.0	-198.5, 230.0	170.0, 413.0	-164.0, 125.5	157.0, 455.0	-198.5, 230.0
Week 52						
n	24	24	16	16	40	40
Mean (SD)	256.46 (59.800)	-54.85 (100.076)	265.63 (68.885)	-11.75 (90.089)	260.13 (62.887)	-37.61 (97.393)
Median	253.00	-32.75	251.50	7.00	253.00	-25.50
Min, Max	155.0, 360.0	-436.5, 48.5	175.0, 455.0	-189.0, 149.0	155.0, 455.0	-436.5, 149.0

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.3

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< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	25	25	15	15	40	40
Mean (SD)	292.00 (105.230)	-20.38 (130.628)	296.53 (118.824)	17.13 (120.102)	293.70 (109.031)	-6.31 (126.559)
Median	280.00	-22.00	256.00	26.00	270.50	-17.50
Min, Max	153.0, 635.0	-329.5, 339.0	186.0, 673.0	-149.0, 339.0	153.0, 673.0	-329.5, 339.0
Week 60						
n	21	21	13	13	34	34
Mean (SD)	253.43 (52.492)	-43.14 (73.338)	279.08 (72.019)	3.77 (71.134)	263.24 (60.960)	-25.21 (75.068)
Median	259.00	-23.00	253.00	24.00	256.00	-14.00
Min, Max	161.0, 367.0	-199.5, 75.5	166.0, 444.0	-151.0, 110.0	161.0, 444.0	-199.5, 110.0
Week 64						
n	19	19	11	11	30	30
Mean (SD)	274.53 (63.527)	-27.05 (83.934)	262.73 (74.169)	-2.41 (64.479)	270.20 (66.598)	-18.02 (77.151)
Median	278.00	-16.00	236.00	-8.00	258.00	-14.25
Min, Max	151.0, 366.0	-227.5, 84.0	167.0, 421.0	-131.5, 87.0	151.0, 421.0	-227.5, 87.0

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.3

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< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	16	16	10	10	26	26
Mean (SD)	267.25 (71.716)	-15.69 (85.685)	243.10 (51.313)	-15.15 (73.525)	257.96 (64.632)	-15.48 (79.696)
Median	284.00	-2.50	234.50	1.00	273.50	1.00
Min, Max	137.0, 412.0	-192.0, 99.5	162.0, 343.0	-131.5, 85.0	137.0, 412.0	-192.0, 99.5
Week 72						
n	13	13	9	9	22	22
Mean (SD)	237.77 (50.712)	-43.19 (85.229)	244.44 (37.918)	-10.56 (76.610)	240.50 (45.039)	-29.84 (81.587)
Median	257.00	-23.50	253.00	-16.00	255.00	-18.00
Min, Max	142.0, 303.0	-206.5, 38.5	182.0, 307.0	-138.0, 120.0	142.0, 307.0	-206.5, 120.0
Week 80						
n	11	11	8	8	19	19
Mean (SD)	284.27 (84.107)	-5.68 (111.796)	243.13 (72.292)	-13.13 (39.669)	266.95 (79.988)	-8.82 (87.004)
Median	296.00	-6.00	240.50	-15.00	244.00	-12.50
Min, Max	153.0, 449.0	-162.5, 215.5	171.0, 389.0	-72.5, 52.0	153.0, 449.0	-162.5, 215.5

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.3

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< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	6	6	12	12
Mean (SD)	334.83 (120.619)	67.08 (146.070)	246.17 (28.562)	9.00 (54.388)	290.50 (95.541)	38.04 (109.376)
Median	350.00	33.25	254.50	20.50	267.00	21.25
Min, Max	176.0, 529.0	-91.0, 329.0	211.0, 274.0	-93.5, 59.0	176.0, 529.0	-93.5, 329.0
Week 96						
n	5	5	5	5	10	10
Mean (SD)	257.20 (69.063)	-24.10 (63.972)	254.60 (64.396)	6.00 (51.148)	255.90 (62.966)	-9.05 (56.862)
Median	250.00	-10.50	230.00	-24.00	240.00	-17.25
Min, Max	165.0, 348.0	-105.0, 61.5	178.0, 342.0	-40.5, 77.0	165.0, 348.0	-105.0, 77.0
Week 104						
n	0	0	2	2	2	2
Mean (SD)			256.00 (49.497)	-21.25 (78.135)	256.00 (49.497)	-21.25 (78.135)
Median			256.00	-21.25	256.00	-21.25
Min, Max			221.0, 291.0	-76.5, 34.0	221.0, 291.0	-76.5, 34.0

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.8.3

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		5		21	
Mean (SD)	285.09 (52.717)		241.00 (39.453)		274.60 (52.592)	
Median	274.50		235.50		258.50	
Min, Max	204.0, 393.5		186.5, 294.0		186.5, 393.5	
Week 1						
n	15	15	5	4		
Mean (SD)	233.53 (49.435)	-54.73 (27.400)	435.20 (461.524)	-13.38 (22.488)		
Median	226.00	-52.50	241.00	-11.50		
Min, Max	161.0, 321.0	-94.5, 2.0	198.0, 1260.0	-42.0, 11.5		
Week 2						
n	16	16	6	5		
Mean (SD)	240.50 (68.770)	-44.59 (40.961)	406.00 (419.432)	-5.80 (11.520)		
Median	228.50	-42.75	245.50	0.50		
Min, Max	126.0, 361.0	-114.5, 23.0	192.0, 1260.0	-18.5, 5.5		

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Alexion Pharmaceuticals, Inc.
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≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	16	16	5	4		
Mean (SD)	243.00 (66.348)	-42.09 (39.435)	434.40 (462.361)	-14.38 (24.428)		
Median	243.50	-43.75	244.00	-5.00		
Min, Max	121.0, 344.0	-119.5, 24.0	187.0, 1260.0	-50.0, 2.5		
Week 4						
n	16	16	6	5		
Mean (SD)	265.69 (82.238)	-19.41 (49.479)	421.50 (412.014)	12.80 (35.006)		
Median	283.00	-0.50	265.00	14.50		
Min, Max	108.0, 389.0	-132.5, 45.5	200.0, 1260.0	-40.0, 58.5		
Week 6						
n	16	16	6	5		
Mean (SD)	266.25 (75.805)	-18.84 (42.227)	406.33 (419.225)	-5.40 (29.687)		
Median	269.50	-8.00	249.00	1.50		
Min, Max	124.0, 405.0	-116.5, 34.5	188.0, 1260.0	-54.0, 22.5		

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.8.3

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	16	16	6	5		
Mean (SD)	286.94 (99.220)	1.84 (62.605)	413.33 (415.774)	3.00 (28.456)		
Median	288.00	1.75	261.00	0.50		
Min, Max	145.0, 482.0	-95.5, 123.0	204.0, 1260.0	-31.0, 42.5		
Week 10						
n	15	15	6	5		
Mean (SD)	282.73 (64.118)	4.87 (42.624)	395.50 (423.836)	-18.40 (21.864)		
Median	285.00	9.00	227.00	-17.50		
Min, Max	174.0, 399.0	-63.0, 82.5	200.0, 1260.0	-48.0, 13.5		
Week 12						
n	14	14	6	5		
Mean (SD)	280.64 (62.448)	8.07 (50.778)	398.33 (422.708)	-15.00 (19.666)		
Median	306.50	14.50	234.00	-7.50		
Min, Max	142.0, 342.0	-95.0, 99.5	188.0, 1260.0	-49.0, 1.5		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.8.3

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	14	14	6	5		
Mean (SD)	310.07 (180.013)	32.25 (178.586)	311.83 (266.914)	-37.60 (42.735)		
Median	280.50	-9.75	212.50	-24.50		
Min, Max	129.0, 882.0	-111.5, 627.5	162.0, 854.0	-103.0, 3.5		
Week 14						
n	14	14	6	5		
Mean (SD)	285.57 (96.627)	13.00 (90.471)	258.83 (123.330)	-30.60 (42.271)		
Median	286.00	-5.25	223.50	-12.50		
Min, Max	153.0, 487.0	-87.5, 208.5	158.0, 501.0	-104.0, -1.5		
Week 15						
n	15	15	6	5		
Mean (SD)	269.80 (76.994)	-8.07 (60.339)	242.67 (65.473)	-22.00 (42.282)		
Median	276.00	-18.00	239.50	-19.50		
Min, Max	155.0, 444.0	-85.5, 165.5	167.0, 361.0	-92.0, 11.5		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.8.3

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	15	15	6	5		
Mean (SD)	319.60 (129.650)	41.73 (114.469)	239.33 (48.007)	-17.40 (52.539)		
Median	328.00	26.00	239.50	-4.50		
Min, Max	172.0, 639.0	-65.0, 332.0	186.0, 318.0	-108.0, 25.5		
Week 18						
n	14	14	6	5		
Mean (SD)	268.21 (66.170)	-4.36 (58.640)	261.33 (38.800)	10.80 (63.490)		
Median	278.00	-6.75	268.00	25.50		
Min, Max	170.0, 392.0	-73.0, 113.5	200.0, 309.0	-94.0, 67.5		
Week 20						
n	13	13	6	5		
Mean (SD)	273.00 (60.932)	-8.58 (48.506)	267.17 (68.034)	10.00 (73.389)		
Median	263.00	-23.50	280.50	14.50		
Min, Max	168.0, 400.0	-72.5, 89.5	185.0, 348.0	-109.0, 84.5		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.8.3

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	15	15	6	5		
Mean (SD)	259.67 (70.975)	-18.20 (49.760)	273.33 (52.823)	20.60 (71.474)		
Median	268.00	-16.00	279.50	51.50		
Min, Max	147.0, 405.0	-93.5, 78.5	199.0, 332.0	-95.0, 83.5		
Week 24						
n	15	15	6	5		
Mean (SD)	290.00 (72.749)	12.13 (59.981)	278.83 (78.341)	23.80 (83.694)		
Median	280.00	6.00	284.50	6.50		
Min, Max	170.0, 468.0	-70.5, 142.0	183.0, 366.0	-88.0, 135.5		
Week 28						
n	14	14	6	5	20	19
Mean (SD)	251.36 (55.685)	-21.21 (46.636)	284.33 (63.971)	33.20 (98.375)	261.25 (58.642)	-6.89 (65.783)
Median	260.50	-25.50	308.00	27.50	276.00	-15.50
Min, Max	156.0, 322.0	-83.0, 79.5	193.0, 342.0	-101.0, 143.5	156.0, 342.0	-101.0, 143.5

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	14	14	5	4	19	18
Mean (SD)	278.14 (65.018)	-0.25 (41.367)	248.20 (79.478)	-22.88 (59.287)	270.26 (68.120)	-5.28 (44.973)
Median	307.00	1.00	259.00	-6.00	279.00	1.00
Min, Max	161.0, 366.0	-79.5, 67.5	154.0, 358.0	-103.0, 23.5	154.0, 366.0	-103.0, 67.5
Week 36						
n	15	15	5	4	20	19
Mean (SD)	300.40 (152.528)	22.53 (136.796)	259.80 (65.842)	-0.88 (51.201)	290.25 (135.574)	17.61 (122.832)
Median	277.00	-14.50	256.00	0.00	271.50	-14.50
Min, Max	168.0, 804.0	-86.0, 470.0	166.0, 328.0	-61.0, 57.5	166.0, 804.0	-86.0, 470.0
Week 40						
n	13	13	5	4	18	17
Mean (SD)	295.77 (84.021)	17.92 (68.824)	269.00 (85.320)	3.38 (90.411)	288.33 (82.754)	14.50 (71.594)
Median	297.00	16.00	302.00	18.00	299.50	16.00
Min, Max	170.0, 516.0	-70.5, 209.0	177.0, 357.0	-117.0, 94.5	170.0, 516.0	-117.0, 209.0

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	13	13	4	3	17	16
Mean (SD)	275.69 (58.344)	-2.15 (43.096)	268.75 (67.771)	-13.67 (91.149)	274.06 (58.510)	-4.31 (51.138)
Median	304.00	-8.00	285.50	26.50	304.00	-5.50
Min, Max	181.0, 329.0	-59.5, 66.5	176.0, 328.0	-118.0, 50.5	176.0, 329.0	-118.0, 66.5
Week 48						
n	11	11	4	3	15	14
Mean (SD)	292.82 (62.830)	13.91 (54.794)	281.50 (76.548)	-5.67 (89.480)	289.80 (64.048)	9.71 (60.090)
Median	298.00	5.50	289.00	7.50	298.00	6.50
Min, Max	180.0, 405.0	-60.5, 126.5	193.0, 355.0	-101.0, 76.5	180.0, 405.0	-101.0, 126.5
Week 52						
n	11	11	3	2	14	13
Mean (SD)	284.45 (63.127)	5.55 (44.432)	287.00 (72.808)	-18.25 (68.943)	285.00 (62.307)	1.88 (46.056)
Median	283.00	2.00	266.00	-18.25	280.50	2.00
Min, Max	199.0, 387.0	-43.5, 82.5	227.0, 368.0	-67.0, 30.5	199.0, 387.0	-67.0, 82.5

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.8.3

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	11	11	3	2	14	13
Mean (SD)	306.73 (69.540)	27.82 (58.146)	315.00 (67.550)	17.25 (9.546)	308.50 (66.590)	26.19 (53.299)
Median	321.00	3.00	318.00	17.25	319.50	10.50
Min, Max	199.0, 439.0	-41.5, 168.5	246.0, 381.0	10.5, 24.0	199.0, 439.0	-41.5, 168.5
Week 60						
n	10	10	3	2	13	12
Mean (SD)	287.40 (85.006)	13.10 (85.262)	264.33 (102.344)	-56.25 (88.742)	282.08 (85.250)	1.54 (85.980)
Median	289.50	1.00	242.00	-56.25	283.00	-2.00
Min, Max	168.0, 444.0	-74.0, 204.0	175.0, 376.0	-119.0, 6.5	168.0, 444.0	-119.0, 204.0
Week 64						
n	9	9	3	2	12	11
Mean (SD)	282.56 (44.450)	4.50 (43.157)	266.33 (107.714)	-60.25 (57.629)	278.50 (60.002)	-7.27 (50.082)
Median	299.00	-1.00	216.00	-60.25	287.00	-15.00
Min, Max	202.0, 334.0	-64.0, 61.5	193.0, 390.0	-101.0, -19.5	193.0, 390.0	-101.0, 61.5

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≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	7	7	2	2	9	9
Mean (SD)	277.43 (17.482)	7.43 (32.842)	215.50 (61.518)	-49.25 (102.884)	263.67 (38.053)	-5.17 (52.505)
Median	275.00	3.50	215.50	-49.25	273.00	3.50
Min, Max	258.0, 306.0	-48.0, 51.5	172.0, 259.0	-122.0, 23.5	172.0, 306.0	-122.0, 51.5
Week 72						
n	6	6	2	2	8	8
Mean (SD)	298.00 (67.186)	36.33 (42.655)	198.00 (50.912)	-66.75 (92.277)	273.00 (75.746)	10.56 (69.232)
Median	290.50	49.25	198.00	-66.75	252.50	13.75
Min, Max	228.0, 403.0	-18.5, 78.0	162.0, 234.0	-132.0, -1.5	162.0, 403.0	-132.0, 78.0
Week 80						
n	6	6	0	0	6	6
Mean (SD)	263.83 (38.974)	2.17 (45.683)			263.83 (38.974)	2.17 (45.683)
Median	276.50	0.50			276.50	0.50
Min, Max	214.0, 304.0	-51.0, 61.5			214.0, 304.0	-51.0, 61.5

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≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	5	5	1	1	6	6
Mean (SD)	270.40 (24.846)	21.40 (16.009)	219.00 (NA)	-16.50 (NA)	261.83 (30.564)	15.08 (21.082)
Median	277.00	21.50	219.00	-16.50	263.50	15.75
Min, Max	239.0, 294.0	1.5, 39.5	219.0, 219.0	-16.5, -16.5	219.0, 294.0	-16.5, 39.5
Week 96						
n	2	2	1	1	3	3
Mean (SD)	233.00 (18.385)	-5.75 (16.617)	183.00 (NA)	-52.50 (NA)	216.33 (31.660)	-21.33 (29.438)
Median	233.00	-5.75	183.00	-52.50	220.00	-17.50
Min, Max	220.0, 246.0	-17.5, 6.0	183.0, 183.0	-52.5, -52.5	183.0, 246.0	-52.5, 6.0
Week 104						
n	1	1	0	0	1	1
Mean (SD)	193.00 (NA)	-44.50 (NA)			193.00 (NA)	-44.50 (NA)
Median	193.00	-44.50			193.00	-44.50
Min, Max	193.0, 193.0	-44.5, -44.5			193.0, 193.0	-44.5, -44.5

Baseline is defined as the average of all available assessments prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Source: ADaM.ADSL, ADaM.ADLB
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Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region Modified Randomized Set

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		13		37	
Mean (SD)	301.53 (155.426)		275.62 (64.127)		292.43 (130.238)	
Median	250.00		247.00		247.00	
Min, Max	165.5, 809.0		187.0, 372.0		165.5, 809.0	
Week 1						
n	22	21	12	12		
Mean (SD)	253.59 (101.661)	-65.59 (86.541)	283.75 (73.557)	4.79 (67.668)		
Median	225.50	-49.50	253.50	-2.50		
Min, Max	151.0, 532.0	-295.5, 87.0	194.0, 404.0	-89.0, 155.0		
Week 2						
n	25	24	13	13		
Mean (SD)	265.40 (170.585)	-64.78 (78.936)	338.69 (263.704)	63.08 (273.440)		
Median	222.00	-55.50	262.00	-3.50		
Min, Max	126.0, 953.0	-294.0, 20.5	179.0, 1195.0	-155.0, 955.0		

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Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:23

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2
and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	23	22	12	12		
Mean (SD)	233.26 (91.198)	-75.00 (92.137)	283.50 (91.592)	4.54 (105.146)		
Median	216.00	-53.50	267.50	-6.25		
Min, Max	121.0, 540.0	-398.5, 9.5	149.0, 433.0	-185.0, 193.0		
Week 4						
n	25	24	10	10		
Mean (SD)	277.96 (159.669)	-47.28 (80.018)	278.50 (64.246)	14.35 (43.808)		
Median	231.00	-16.75	273.00	19.50		
Min, Max	108.0, 847.0	-259.5, 45.5	178.0, 389.0	-82.5, 76.5		
Week 6						
n	23	22	13	13		
Mean (SD)	296.35 (168.364)	-17.20 (59.827)	316.23 (169.581)	40.62 (188.119)		
Median	259.00	-26.25	264.00	22.50		
Min, Max	124.0, 794.0	-116.5, 142.0	167.0, 843.0	-167.0, 603.0		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region Modified Randomized Set

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	24	23	12	12		
Mean (SD)	279.71 (110.691)	-29.45 (103.088)	312.42 (126.812)	44.83 (133.727)		
Median	259.00	-28.00	276.50	13.75		
Min, Max	145.0, 543.0	-337.5, 123.0	180.0, 672.0	-113.5, 432.0		
Week 10						
n	23	22	13	13		
Mean (SD)	262.04 (91.184)	-16.99 (60.719)	283.08 (86.633)	7.46 (98.160)		
Median	238.00	-16.00	259.00	8.50		
Min, Max	148.0, 589.0	-220.0, 82.5	168.0, 459.0	-137.0, 191.5		
Week 12						
n	21	20	13	13		
Mean (SD)	264.05 (87.096)	-31.79 (117.423)	285.23 (69.435)	9.62 (45.165)		
Median	272.00	-10.00	272.00	12.00		
Min, Max	135.0, 519.0	-379.5, 99.5	179.0, 394.0	-84.5, 80.0		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region Modified Randomized Set

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	22	21	12	12		
Mean (SD)	266.41 (111.250)	-34.71 (124.789)	210.42 (39.775)	-58.46 (69.301)		
Median	245.00	-18.00	221.00	-36.50		
Min, Max	129.0, 639.0	-496.5, 133.5	138.0, 259.0	-196.0, 12.0		
Week 14						
n	22	21	12	12		
Mean (SD)	268.86 (99.997)	-29.97 (127.399)	223.58 (37.747)	-47.17 (72.178)		
Median	265.50	-13.00	220.50	-9.25		
Min, Max	153.0, 587.0	-442.5, 208.5	182.0, 322.0	-189.0, 16.0		
Week 15						
n	21	20	11	11		
Mean (SD)	272.29 (115.514)	-29.18 (136.969)	213.27 (37.855)	-54.32 (66.461)		
Median	250.00	-8.75	219.00	-33.00		
Min, Max	134.0, 627.0	-486.5, 209.5	154.0, 276.0	-168.0, 29.0		

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 Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region Modified Randomized Set

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	24	23	13	13		
Mean (SD)	291.67 (115.433)	-6.10 (108.602)	255.54 (69.597)	-20.08 (106.018)		
Median	271.00	-6.50	255.00	-13.00		
Min, Max	144.0, 582.0	-258.5, 243.5	152.0, 439.0	-182.0, 252.0		
Week 18						
n	23	22	13	13		
Mean (SD)	281.39 (104.745)	-14.02 (101.077)	271.85 (79.603)	-3.77 (56.333)		
Median	269.00	-9.42	239.00	-12.00		
Min, Max	163.0, 568.0	-297.5, 113.5	175.0, 461.0	-111.0, 93.5		
Week 20						
n	20	19	13	13		
Mean (SD)	294.05 (132.677)	-16.07 (72.309)	278.15 (87.544)	2.54 (81.834)		
Median	264.00	-14.50	250.00	1.00		
Min, Max	157.0, 725.0	-228.5, 89.5	190.0, 535.0	-100.5, 229.0		

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Post-hoc Analysen Studie ALPHA

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ALXN2040-PNH-301

Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region Modified Randomized Set

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	24	23	13	13		
Mean (SD)	278.58 (157.538)	-22.41 (87.279)	269.69 (58.061)	-5.92 (74.247)		
Median	248.50	-18.50	262.00	0.50		
Min, Max	139.0, 906.0	-307.0, 171.5	184.0, 416.0	-145.0, 110.0		
Week 24						
n	23	22	13	13		
Mean (SD)	261.22 (89.136)	-41.22 (128.478)	272.00 (63.314)	-3.62 (83.561)		
Median	256.00	-18.50	251.00	6.50		
Min, Max	130.0, 506.0	-491.5, 116.0	188.0, 442.0	-125.0, 136.0		
Week 28						
n	20	20	13	13	33	33
Mean (SD)	248.00 (95.365)	-51.69 (119.311)	269.92 (66.759)	-5.69 (85.274)	256.64 (84.790)	-33.57 (108.166)
Median	240.00	-28.00	254.00	1.00	246.00	-15.50
Min, Max	134.0, 591.0	-467.5, 83.7	173.0, 433.0	-161.0, 127.0	134.0, 591.0	-467.5, 127.0

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Post-hoc Analysen Studie ALPHA

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Europe

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	19	18	13	13	32	31
Mean (SD)	282.21 (102.152)	-37.33 (111.552)	265.69 (70.486)	-9.92 (99.156)	275.50 (89.723)	-25.84 (105.704)
Median	270.00	-10.75	250.00	-1.00	257.50	-6.00
Min, Max	150.0, 512.0	-331.5, 101.0	192.0, 421.0	-167.0, 153.5	150.0, 512.0	-331.5, 153.5
Week 36						
n	18	17	11	11	29	28
Mean (SD)	231.28 (53.423)	-20.05 (53.446)	279.91 (85.532)	17.95 (69.701)	249.72 (70.158)	-5.12 (62.043)
Median	229.00	-34.50	256.00	3.00	256.00	-20.50
Min, Max	131.0, 318.0	-86.0, 111.7	172.0, 478.0	-62.5, 172.0	131.0, 478.0	-86.0, 172.0
Week 40						
n	18	17	12	12	30	29
Mean (SD)	257.44 (50.529)	-22.20 (102.985)	278.33 (67.616)	0.33 (74.565)	265.80 (57.786)	-12.87 (91.500)
Median	257.00	0.67	261.50	-19.75	257.00	-6.00
Min, Max	170.0, 339.0	-395.5, 54.5	170.0, 394.0	-120.0, 120.0	170.0, 394.0	-395.5, 120.0

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Run Date: 2024-04-26T15:50:23

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Post-hoc Analysen Studie ALPHA

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Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	17	16	12	12	29	28
Mean (SD)	242.94 (57.242)	-37.24 (116.428)	263.08 (53.505)	-4.50 (69.010)	251.28 (55.669)	-23.21 (98.708)
Median	255.00	-21.75	257.00	3.00	255.00	-18.50
Min, Max	149.0, 318.0	-443.5, 78.0	164.0, 356.0	-115.5, 81.0	149.0, 356.0	-443.5, 81.0
Week 48						
n	12	12	13	13	25	25
Mean (SD)	270.17 (73.208)	18.38 (60.569)	262.54 (60.550)	-13.08 (62.075)	266.20 (65.610)	2.02 (62.171)
Median	275.00	21.50	265.00	-9.50	265.00	5.50
Min, Max	157.0, 405.0	-67.5, 126.5	170.0, 351.0	-164.0, 62.0	157.0, 405.0	-164.0, 126.5
Week 52						
n	12	12	13	13	25	25
Mean (SD)	244.00 (55.324)	-44.33 (129.416)	267.00 (72.435)	-8.62 (92.092)	255.96 (64.527)	-25.76 (110.673)
Median	238.00	-30.00	247.00	-4.00	242.00	-24.00
Min, Max	155.0, 327.0	-436.5, 82.5	175.0, 455.0	-189.0, 149.0	155.0, 455.0	-436.5, 149.0

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.8.4

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Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	13	13	12	12	25	25
Mean (SD)	319.38 (130.683)	30.46 (158.330)	304.75 (131.061)	26.75 (125.227)	312.36 (128.326)	28.68 (140.447)
Median	295.00	-4.00	262.50	-2.75	272.00	-4.00
Min, Max	153.0, 635.0	-329.5, 339.0	186.0, 673.0	-125.0, 339.0	153.0, 673.0	-329.5, 339.0
Week 60						
n	10	10	10	10	20	20
Mean (SD)	237.60 (55.837)	-2.45 (45.005)	283.80 (78.256)	11.40 (61.393)	260.70 (70.281)	4.48 (52.870)
Median	239.00	4.00	265.50	-1.25	245.00	1.00
Min, Max	161.0, 313.0	-72.5, 60.5	166.0, 444.0	-67.5, 110.0	161.0, 444.0	-72.5, 110.0
Week 64						
n	9	9	8	8	17	17
Mean (SD)	247.33 (59.150)	7.33 (46.254)	241.38 (75.832)	-16.31 (62.844)	244.53 (65.381)	-3.79 (54.273)
Median	242.00	-14.50	222.50	-16.75	228.00	-14.50
Min, Max	151.0, 334.0	-52.0, 68.0	167.0, 421.0	-131.5, 87.0	151.0, 421.0	-131.5, 87.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2x$ ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region Modified Randomized Set

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	8	8	7	7	15	15
Mean (SD)	258.13 (48.880)	22.94 (45.364)	232.43 (40.054)	-14.36 (73.186)	246.13 (45.368)	5.53 (60.789)
Median	274.00	19.50	236.00	-12.00	259.00	17.50
Min, Max	143.0, 294.0	-44.0, 93.0	162.0, 272.0	-131.5, 85.0	143.0, 294.0	-131.5, 93.0
Week 72						
n	7	7	7	7	14	14
Mean (SD)	242.29 (69.689)	10.29 (44.012)	245.57 (38.118)	-1.21 (67.690)	243.93 (53.991)	4.54 (55.176)
Median	228.00	-9.50	245.00	-16.00	239.50	-12.75
Min, Max	142.0, 340.0	-30.0, 69.5	182.0, 307.0	-103.5, 120.0	142.0, 340.0	-103.5, 120.0
Week 80						
n	6	6	5	5	11	11
Mean (SD)	253.33 (64.398)	18.33 (64.922)	226.80 (49.317)	-22.60 (47.396)	241.27 (56.907)	-0.27 (58.847)
Median	264.50	6.00	239.00	-25.50	239.00	-15.00
Min, Max	153.0, 320.0	-60.0, 120.0	171.0, 295.0	-72.5, 52.0	153.0, 320.0	-72.5, 120.0

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	5	5	11	11
Mean (SD)	310.50 (121.630)	75.50 (125.651)	244.20 (28.926)	-7.70 (55.435)	280.36 (94.501)	37.68 (104.934)
Median	284.50	28.00	246.00	3.50	271.00	10.50
Min, Max	176.0, 529.0	1.5, 329.0	211.0, 274.0	-93.5, 59.0	176.0, 529.0	-93.5, 329.0
Week 96						
n	3	3	5	5	8	8
Mean (SD)	204.33 (34.298)	-28.00 (33.989)	232.00 (66.042)	-19.90 (37.053)	221.63 (55.076)	-22.94 (33.648)
Median	220.00	-17.50	227.00	-25.50	223.50	-24.75
Min, Max	165.0, 228.0	-66.0, -0.5	178.0, 342.0	-52.5, 43.0	165.0, 342.0	-66.0, 43.0
Week 104						
n	1	1	2	2	3	3
Mean (SD)	193.00 (NA)	-44.50 (NA)	256.00 (49.497)	-21.25 (78.135)	235.00 (50.478)	-29.00 (56.857)
Median	193.00	-44.50	256.00	-21.25	221.00	-44.50
Min, Max	193.0, 193.0	-44.5, -44.5	221.0, 291.0	-76.5, 34.0	193.0, 291.0	-76.5, 34.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	305.84 (95.775)		283.83 (105.430)		299.84 (97.749)	
Median	291.00		273.00		281.25	
Min, Max	140.0, 479.5		139.0, 506.0		139.0, 506.0	
Week 1						
n	29	29	13	12		
Mean (SD)	241.90 (62.778)	-65.83 (62.555)	317.31 (290.247)	-45.08 (92.807)		
Median	241.00	-46.50	241.00	-18.25		
Min, Max	137.0, 396.0	-243.5, 9.5	128.0, 1260.0	-332.0, 13.0		
Week 2						
n	32	32	13	12		
Mean (SD)	234.59 (59.933)	-71.25 (80.305)	319.08 (291.158)	-43.17 (98.759)		
Median	232.50	-44.50	232.00	-9.00		
Min, Max	117.0, 354.0	-307.5, 66.5	144.0, 1260.0	-345.0, 5.5		

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Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	31	31	12	11		
Mean (SD)	230.26 (63.401)	-77.79 (79.213)	322.58 (301.888)	-26.27 (28.015)		
Median	230.00	-58.00	237.00	-16.00		
Min, Max	122.0, 370.0	-301.5, 24.0	123.0, 1260.0	-83.0, 2.5		
Week 4						
n	31	31	12	11		
Mean (SD)	253.68 (65.384)	-51.21 (79.192)	338.92 (298.121)	-8.45 (20.542)		
Median	259.00	-40.00	261.00	-15.00		
Min, Max	138.0, 430.0	-224.5, 114.0	123.0, 1260.0	-40.0, 17.5		
Week 6						
n	31	31	12	11		
Mean (SD)	255.94 (52.196)	-52.24 (78.569)	348.25 (304.867)	1.73 (44.296)		
Median	250.00	-47.00	240.50	-4.00		
Min, Max	146.0, 359.0	-238.5, 78.0	130.0, 1260.0	-54.0, 98.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	30	30	12	11		
Mean (SD)	287.03 (100.456)	-18.63 (72.474)	356.42 (299.663)	10.64 (38.747)		
Median	254.50	-19.75	261.00	10.00		
Min, Max	146.0, 629.0	-160.0, 255.5	122.0, 1260.0	-43.0, 92.5		
Week 10						
n	28	28	12	11		
Mean (SD)	263.89 (57.847)	-40.39 (78.467)	335.75 (300.145)	-11.91 (28.952)		
Median	263.50	-18.50	234.50	-4.00		
Min, Max	158.0, 365.0	-213.0, 55.0	155.0, 1260.0	-67.5, 16.0		
Week 12						
n	31	31	12	11		
Mean (SD)	266.32 (59.173)	-37.34 (75.655)	337.00 (300.111)	-10.55 (28.383)		
Median	251.00	-14.00	247.50	-7.50		
Min, Max	171.0, 453.0	-230.5, 53.0	128.0, 1260.0	-54.5, 31.0		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	31	31	11	10		
Mean (SD)	276.00 (126.220)	-27.66 (145.783)	269.73 (200.889)	-42.00 (52.320)		
Median	253.00	-6.00	219.00	-20.75		
Min, Max	146.0, 882.0	-259.5, 627.5	122.0, 854.0	-128.0, 27.5		
Week 14						
n	31	31	11	10		
Mean (SD)	276.19 (92.531)	-27.47 (103.044)	245.64 (103.718)	-33.20 (44.856)		
Median	254.00	-18.00	224.00	-18.00		
Min, Max	135.0, 573.0	-243.5, 214.5	118.0, 501.0	-104.0, 22.5		
Week 15						
n	30	30	11	10		
Mean (SD)	266.03 (76.320)	-40.25 (93.563)	225.91 (65.453)	-40.90 (44.676)		
Median	254.00	-26.75	221.00	-20.50		
Min, Max	142.0, 521.0	-243.5, 283.5	123.0, 361.0	-127.0, 11.5		

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	30	30	11	10		
Mean (SD)	279.77 (97.522)	-23.28 (105.209)	235.09 (74.231)	-26.50 (47.298)		
Median	260.50	-20.50	241.00	-16.00		
Min, Max	159.0, 639.0	-239.5, 332.0	121.0, 390.0	-108.0, 26.0		
Week 18						
n	31	31	10	9		
Mean (SD)	256.19 (54.363)	-47.47 (75.364)	245.20 (59.529)	-11.39 (60.678)		
Median	254.00	-8.00	268.00	2.00		
Min, Max	159.0, 374.0	-222.5, 33.0	141.0, 309.0	-105.0, 67.5		
Week 20						
n	29	29	11	10		
Mean (SD)	273.69 (91.281)	-36.03 (89.713)	251.73 (70.600)	-11.20 (64.323)		
Median	261.00	-29.00	269.00	-1.50		
Min, Max	171.0, 630.0	-243.5, 235.5	137.0, 348.0	-127.0, 84.5		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	30	30	11	10		
Mean (SD)	259.17 (63.230)	-48.88 (75.523)	260.73 (62.471)	0.30 (79.897)		
Median	260.50	-31.50	256.00	22.50		
Min, Max	108.0, 363.0	-224.5, 74.5	169.0, 349.0	-180.0, 83.5		
Week 24						
n	29	29	10	9		
Mean (SD)	288.41 (94.145)	-22.50 (87.587)	291.90 (77.541)	19.56 (79.531)		
Median	277.00	-14.00	307.50	45.00		
Min, Max	139.0, 573.0	-201.5, 142.0	183.0, 394.0	-116.0, 135.5		
Week 28						
n	31	31	10	9	41	40
Mean (SD)	287.48 (91.413)	-16.18 (105.611)	290.80 (70.381)	19.89 (84.453)	288.29 (85.929)	-8.06 (101.368)
Median	274.00	-10.00	305.00	22.50	277.00	-7.25
Min, Max	173.0, 632.0	-208.0, 370.5	193.0, 405.0	-104.0, 143.5	173.0, 632.0	-208.0, 370.5

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	29	29	9	8	38	37
Mean (SD)	275.52 (78.249)	-29.79 (79.011)	284.67 (92.878)	5.06 (91.340)	277.68 (80.711)	-22.26 (81.789)
Median	250.00	-7.00	267.00	21.25	250.00	-6.00
Min, Max	173.0, 467.0	-255.5, 79.5	154.0, 421.0	-137.0, 133.5	154.0, 467.0	-255.5, 133.5
Week 36						
n	29	29	9	8	38	37
Mean (SD)	293.69 (115.500)	-17.40 (125.620)	284.44 (79.609)	8.56 (55.630)	291.50 (107.152)	-11.78 (113.986)
Median	256.00	-5.00	282.00	-3.75	266.50	-5.00
Min, Max	193.0, 804.0	-229.5, 470.0	166.0, 426.0	-61.0, 92.0	166.0, 804.0	-229.5, 470.0
Week 40						
n	28	28	9	8	37	36
Mean (SD)	299.93 (96.406)	-14.98 (106.355)	262.56 (72.538)	-19.69 (79.402)	290.84 (91.674)	-16.03 (99.954)
Median	277.50	-9.25	265.00	6.25	271.00	-8.75
Min, Max	201.0, 623.0	-230.5, 209.0	177.0, 376.0	-164.0, 46.0	177.0, 623.0	-230.5, 209.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	26	26	8	7	34	33
Mean (SD)	274.65 (54.351)	-39.65 (66.362)	266.13 (68.347)	-25.14 (88.252)	272.65 (56.941)	-36.58 (70.265)
Median	269.00	-25.00	261.00	17.00	269.00	-19.50
Min, Max	181.0, 394.0	-210.5, 93.0	176.0, 373.0	-180.0, 50.5	176.0, 394.0	-210.5, 93.0
Week 48						
n	24	24	7	6	31	30
Mean (SD)	289.92 (72.865)	-20.23 (85.693)	293.71 (82.520)	9.67 (89.603)	290.77 (73.722)	-14.25 (85.769)
Median	277.00	-14.50	316.00	22.50	279.00	-12.25
Min, Max	173.0, 455.0	-198.5, 230.0	193.0, 413.0	-101.0, 125.5	173.0, 455.0	-198.5, 230.0
Week 52						
n	23	23	6	5	29	28
Mean (SD)	276.35 (62.557)	-31.46 (64.691)	273.33 (62.940)	-22.50 (77.622)	275.72 (61.512)	-29.86 (65.687)
Median	278.00	-23.00	261.00	18.00	266.00	-19.50
Min, Max	184.0, 387.0	-170.5, 67.0	198.0, 368.0	-138.0, 37.5	184.0, 387.0	-170.5, 67.0

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	23	23	6	5	29	28
Mean (SD)	283.57 (67.481)	-26.07 (77.964)	289.33 (59.581)	-5.90 (80.269)	284.76 (64.942)	-22.46 (77.260)
Median	282.00	-14.50	285.50	26.00	282.00	0.25
Min, Max	161.0, 425.0	-233.5, 111.5	221.0, 381.0	-149.0, 41.0	161.0, 425.0	-233.5, 111.5
Week 60						
n	21	21	6	5	27	26
Mean (SD)	277.14 (66.824)	-35.74 (91.839)	263.83 (73.633)	-35.50 (91.632)	274.19 (67.152)	-35.69 (89.950)
Median	280.00	-38.00	248.00	24.00	261.00	-30.50
Min, Max	181.0, 444.0	-199.5, 204.0	175.0, 376.0	-151.0, 35.5	175.0, 444.0	-199.5, 204.0
Week 64						
n	19	19	6	5	25	24
Mean (SD)	291.21 (52.226)	-28.39 (82.623)	293.00 (75.966)	-3.30 (74.819)	291.64 (56.996)	-23.17 (80.153)
Median	307.00	-15.00	299.50	43.00	307.00	-10.75
Min, Max	186.0, 366.0	-227.5, 84.0	193.0, 390.0	-101.0, 57.5	186.0, 390.0	-227.5, 84.0

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	15	15	5	5	20	20
Mean (SD)	276.87 (65.970)	-25.50 (81.309)	247.00 (68.018)	-29.90 (84.165)	269.40 (66.006)	-26.60 (79.791)
Median	284.00	-14.00	233.00	14.00	284.00	-5.25
Min, Max	137.0, 412.0	-192.0, 99.5	172.0, 343.0	-122.0, 55.5	137.0, 412.0	-192.0, 99.5
Week 72						
n	12	12	4	4	16	16
Mean (SD)	265.25 (57.881)	-34.63 (96.148)	219.25 (48.795)	-55.00 (92.700)	253.75 (57.933)	-39.72 (92.633)
Median	267.50	6.25	224.50	-58.00	261.50	6.25
Min, Max	191.0, 403.0	-206.5, 78.0	162.0, 266.0	-138.0, 34.0	162.0, 403.0	-206.5, 78.0
Week 80						
n	11	11	3	3	14	14
Mean (SD)	290.00 (73.486)	-14.50 (105.070)	270.33 (107.937)	2.67 (20.404)	285.79 (77.566)	-10.82 (92.788)
Median	279.00	-6.00	244.00	-2.00	276.50	-4.00
Min, Max	188.0, 449.0	-162.5, 215.5	178.0, 389.0	-15.0, 25.0	178.0, 449.0	-162.5, 215.5

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.4

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	5	5	2	2	7	7
Mean (SD)	299.60 (55.324)	11.30 (76.186)	237.50 (36.062)	38.00 (8.485)	281.86 (56.351)	18.93 (63.650)
Median	294.00	10.00	237.50	38.00	263.00	32.00
Min, Max	242.0, 362.0	-91.0, 116.5	212.0, 263.0	32.0, 44.0	212.0, 362.0	-91.0, 116.5
Week 96						
n	4	4	1	1	5	5
Mean (SD)	284.75 (47.661)	-12.00 (69.228)	296.00 (NA)	77.00 (NA)	287.00 (41.581)	5.80 (71.962)
Median	272.50	-2.25	296.00	77.00	295.00	6.00
Min, Max	246.0, 348.0	-105.0, 61.5	296.0, 296.0	77.0, 77.0	246.0, 348.0	-105.0, 77.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.5

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		10		34	
Mean (SD)	321.37 (160.878)		279.10 (89.207)		308.94 (143.496)	
Median	253.25		270.50		253.25	
Min, Max	165.5, 809.0		139.0, 404.0		139.0, 809.0	
Week 1						
n	18	18	10	10		
Mean (SD)	268.17 (105.148)	-71.41 (92.131)	269.50 (89.955)	-9.60 (45.342)		
Median	241.00	-55.25	256.00	-9.50		
Min, Max	151.0, 532.0	-295.5, 87.0	128.0, 404.0	-89.0, 64.0		
Week 2						
n	24	24	10	10		
Mean (SD)	253.00 (96.462)	-68.37 (88.622)	265.30 (90.244)	-13.80 (51.968)		
Median	242.00	-53.75	248.00	-1.00		
Min, Max	117.0, 515.0	-294.0, 66.5	144.0, 397.0	-155.0, 28.5		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:26

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

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ALXN2040-PNH-301

Table GERMANY.2.6.8.5

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	22	22	10	10		
Mean (SD)	251.55 (93.090)	-78.86 (97.254)	260.00 (104.789)	-19.10 (92.589)		
Median	241.50	-48.25	242.00	-11.50		
Min, Max	130.0, 540.0	-398.5, 24.0	123.0, 421.0	-185.0, 176.0		
Week 4						
n	24	24	8	8		
Mean (SD)	269.58 (99.561)	-51.78 (89.385)	255.63 (91.824)	-5.56 (24.712)		
Median	268.50	-26.00	254.00	-4.50		
Min, Max	145.0, 551.0	-259.5, 98.5	123.0, 389.0	-40.0, 27.0		
Week 6						
n	23	23	10	10		
Mean (SD)	303.13 (153.222)	-22.06 (66.665)	260.80 (102.499)	-18.30 (73.807)		
Median	259.00	-6.00	248.50	-3.75		
Min, Max	140.0, 794.0	-166.0, 142.0	130.0, 460.0	-167.0, 56.0		

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.8.5

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	21	21	9	9		
Mean (SD)	304.19 (129.274)	-21.59 (118.525)	273.56 (101.260)	4.78 (27.423)		
Median	274.00	-13.00	268.00	4.50		
Min, Max	149.0, 629.0	-337.5, 255.5	122.0, 456.0	-31.0, 52.0		
Week 10						
n	20	20	10	10		
Mean (SD)	276.80 (98.036)	-21.89 (62.657)	253.00 (84.094)	-26.10 (56.597)		
Median	252.50	-10.00	251.00	8.75		
Min, Max	148.0, 589.0	-220.0, 45.0	155.0, 413.0	-137.0, 16.0		
Week 12						
n	22	22	10	10		
Mean (SD)	282.32 (86.223)	-33.40 (109.740)	272.70 (94.894)	-6.40 (24.866)		
Median	276.50	-2.00	252.50	2.25		
Min, Max	135.0, 519.0	-379.5, 66.0	128.0, 410.0	-49.0, 26.5		

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Post-hoc Analysen Studie ALPHA

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Table GERMANY.2.6.8.5

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> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	21	21	10	10		
Mean (SD)	257.67 (103.499)	-59.83 (114.251)	199.50 (51.874)	-79.60 (71.088)		
Median	238.00	-22.50	200.50	-67.00		
Min, Max	136.0, 639.0	-496.5, 27.0	122.0, 276.0	-196.0, 12.0		
Week 14						
n	22	22	9	9		
Mean (SD)	289.32 (105.291)	-26.40 (134.576)	203.11 (52.753)	-69.89 (69.708)		
Median	269.00	-11.00	196.00	-28.50		
Min, Max	152.0, 587.0	-442.5, 214.5	118.0, 303.0	-189.0, 1.0		
Week 15						
n	21	21	9	9		
Mean (SD)	285.81 (120.780)	-35.12 (141.927)	200.78 (51.754)	-68.00 (70.165)		
Median	248.00	-21.00	202.00	-35.00		
Min, Max	134.0, 627.0	-486.5, 283.5	123.0, 277.0	-168.0, 29.0		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:26

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.5

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	22	22	10	10		
Mean (SD)	306.64 (135.388)	-9.08 (129.620)	228.20 (78.052)	-50.90 (75.620)		
Median	254.50	-7.75	218.00	-18.00		
Min, Max	144.0, 639.0	-258.5, 332.0	121.0, 390.0	-182.0, 50.0		
Week 18						
n	22	22	10	10		
Mean (SD)	284.50 (98.637)	-31.22 (97.265)	264.70 (90.946)	-14.40 (72.397)		
Median	269.50	-0.25	238.50	-11.00		
Min, Max	163.0, 568.0	-297.5, 113.5	141.0, 461.0	-111.0, 93.5		
Week 20						
n	18	18	10	10		
Mean (SD)	295.94 (140.983)	-36.46 (61.529)	241.70 (64.483)	-37.40 (63.103)		
Median	260.50	-28.17	253.00	-20.50		
Min, Max	157.0, 725.0	-228.5, 23.0	137.0, 343.0	-127.0, 71.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	22	22	10	10		
Mean (SD)	290.50 (159.173)	-25.22 (88.345)	236.60 (45.998)	-42.50 (88.809)		
Median	258.00	-17.25	236.00	-29.50		
Min, Max	139.0, 906.0	-307.0, 171.5	169.0, 314.0	-180.0, 67.0		
Week 24						
n	21	21	9	9		
Mean (SD)	276.38 (93.627)	-42.25 (133.387)	249.56 (43.532)	-45.11 (83.069)		
Median	260.00	-4.00	251.00	-88.00		
Min, Max	130.0, 506.0	-491.5, 142.0	183.0, 307.0	-125.0, 62.0		
Week 28						
n	22	22	9	9	31	31
Mean (SD)	278.45 (100.551)	-37.27 (119.663)	251.44 (54.886)	-43.22 (97.866)	270.61 (89.644)	-38.99 (112.183)
Median	261.00	-9.00	254.00	-98.00	255.00	-13.00
Min, Max	134.0, 591.0	-467.5, 101.5	173.0, 330.0	-161.0, 143.5	134.0, 591.0	-467.5, 143.5

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	19	19	9	9	28	28
Mean (SD)	294.53 (101.054)	-33.45 (104.195)	231.67 (67.494)	-63.00 (98.017)	274.32 (95.139)	-42.95 (101.399)
Median	269.00	-6.00	211.00	-103.00	249.50	-12.25
Min, Max	150.0, 512.0	-331.5, 49.0	154.0, 388.0	-167.0, 141.0	150.0, 512.0	-331.5, 141.0
Week 36						
n	19	19	7	7	26	26
Mean (SD)	270.37 (78.965)	-0.70 (60.921)	260.71 (85.873)	-17.93 (56.811)	267.77 (79.237)	-5.34 (59.225)
Median	257.00	-5.00	233.00	-30.00	256.50	-25.25
Min, Max	131.0, 404.0	-69.0, 141.0	166.0, 354.0	-61.0, 107.0	131.0, 404.0	-69.0, 141.0
Week 40						
n	19	19	8	8	27	27
Mean (SD)	280.00 (81.143)	-18.99 (118.780)	228.38 (51.705)	-72.25 (59.125)	264.70 (76.519)	-34.77 (106.409)
Median	271.00	4.50	231.00	-64.25	252.00	-18.00
Min, Max	170.0, 516.0	-395.5, 209.0	170.0, 316.0	-164.0, -7.5	170.0, 516.0	-395.5, 209.0

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.5

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> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	17	17	7	7	24	24
Mean (SD)	268.00 (64.714)	-37.23 (118.580)	224.86 (54.560)	-74.21 (84.257)	255.42 (63.962)	-48.01 (109.219)
Median	291.00	-12.50	224.00	-109.00	258.50	-31.50
Min, Max	149.0, 394.0	-443.5, 78.0	164.0, 328.0	-180.0, 81.0	149.0, 394.0	-443.5, 81.0
Week 48						
n	13	13	8	8	21	21
Mean (SD)	285.77 (79.740)	-1.73 (72.153)	247.75 (67.970)	-60.44 (65.709)	271.29 (76.092)	-24.10 (74.083)
Median	275.00	9.50	245.50	-58.50	265.00	-16.50
Min, Max	157.0, 416.0	-157.0, 126.5	170.0, 351.0	-164.0, 41.0	157.0, 416.0	-164.0, 126.5
Week 52						
n	14	14	8	8	22	22
Mean (SD)	261.43 (65.754)	-55.00 (125.222)	246.63 (56.221)	-61.56 (90.391)	256.05 (61.508)	-57.39 (111.539)
Median	259.50	-32.75	241.50	-54.50	241.50	-36.50
Min, Max	155.0, 387.0	-436.5, 67.0	175.0, 347.0	-189.0, 100.0	155.0, 387.0	-436.5, 100.0

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:26

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	14	14	7	7	21	21
Mean (SD)	303.71 (92.635)	-15.71 (132.054)	322.71 (160.145)	5.79 (164.253)	310.05 (115.568)	-8.55 (139.773)
Median	288.50	-8.00	288.00	-16.00	288.00	-12.50
Min, Max	153.0, 439.0	-329.5, 225.0	186.0, 673.0	-149.0, 339.0	153.0, 673.0	-329.5, 339.0
Week 60						
n	12	12	6	6	18	18
Mean (SD)	287.58 (87.494)	-4.08 (100.455)	266.00 (104.087)	-41.75 (91.707)	280.39 (90.826)	-16.64 (96.628)
Median	296.00	4.00	244.50	-40.75	272.00	-22.50
Min, Max	161.0, 444.0	-161.0, 204.0	166.0, 444.0	-151.0, 110.0	161.0, 444.0	-161.0, 204.0
Week 64						
n	13	13	6	6	19	19
Mean (SD)	279.85 (63.120)	-7.65 (72.464)	265.17 (95.847)	-42.58 (77.408)	275.21 (72.506)	-18.68 (73.779)
Median	299.00	-1.00	236.50	-51.00	289.00	-14.50
Min, Max	151.0, 354.0	-166.0, 76.5	167.0, 421.0	-131.5, 87.0	151.0, 421.0	-166.0, 87.0

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Post-hoc Analysen Studie ALPHA

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> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	11	11	5	5	16	16
Mean (SD)	279.27 (72.548)	7.82 (57.643)	217.60 (50.718)	-84.90 (54.866)	260.00 (71.179)	-21.16 (70.625)
Median	284.00	2.50	233.00	-119.00	274.00	-18.25
Min, Max	143.0, 412.0	-73.0, 99.5	162.0, 285.0	-131.5, -12.0	143.0, 412.0	-131.5, 99.5
Week 72						
n	8	8	5	5	13	13
Mean (SD)	245.25 (62.839)	-10.88 (68.303)	220.60 (47.274)	-81.90 (59.788)	235.77 (56.606)	-38.19 (72.155)
Median	247.00	6.25	229.00	-103.50	229.00	-20.00
Min, Max	142.0, 340.0	-150.0, 69.5	162.0, 266.0	-138.0, -16.0	142.0, 340.0	-150.0, 69.5
Week 80						
n	7	7	3	3	10	10
Mean (SD)	295.00 (95.675)	37.86 (102.666)	290.33 (101.081)	-34.17 (33.198)	293.60 (91.532)	16.25 (92.099)
Median	295.00	24.50	295.00	-15.00	295.00	-13.75
Min, Max	153.0, 449.0	-98.0, 215.5	187.0, 389.0	-72.5, -15.0	153.0, 449.0	-98.0, 215.5

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Post-hoc Analysen Studie ALPHA

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> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	7	2	2	9	9
Mean (SD)	314.00 (114.800)	56.86 (134.251)	242.50 (44.548)	-42.25 (72.478)	298.11 (105.482)	34.83 (126.823)
Median	292.00	10.50	242.50	-42.25	274.00	10.00
Min, Max	176.0, 529.0	-91.0, 329.0	211.0, 274.0	-93.5, 9.0	176.0, 529.0	-93.5, 329.0
Week 96						
n	5	5	2	2	7	7
Mean (SD)	254.80 (70.098)	-11.10 (60.278)	260.00 (115.966)	-24.75 (1.061)	256.29 (74.321)	-15.00 (49.667)
Median	246.00	-0.50	260.00	-24.75	246.00	-17.50
Min, Max	165.0, 348.0	-105.0, 61.5	178.0, 342.0	-25.5, -24.0	165.0, 348.0	-105.0, 61.5
Week 104						
n	1	1	1	1	2	2
Mean (SD)	193.00 (NA)	-44.50 (NA)	291.00 (NA)	-76.50 (NA)	242.00 (69.296)	-60.50 (22.627)
Median	193.00	-44.50	291.00	-76.50	242.00	-60.50
Min, Max	193.0, 193.0	-44.5, -44.5	291.0, 291.0	-76.5, -76.5	193.0, 291.0	-76.5, -44.5

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Post-hoc Analysen Studie ALPHA

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		15		47	
Mean (SD)	290.97 (86.561)		279.87 (84.690)		287.43 (85.204)	
Median	268.25		258.50		261.50	
Min, Max	140.0, 479.5		180.0, 506.0		140.0, 506.0	
Week 1						
n	33	32	15	14		
Mean (SD)	235.36 (63.395)	-62.53 (60.681)	322.33 (265.638)	-27.68 (103.638)		
Median	225.00	-44.50	243.00	-18.25		
Min, Max	137.0, 457.0	-243.5, 33.0	174.0, 1260.0	-332.0, 155.0		
Week 2						
n	33	32	16	15		
Mean (SD)	244.55 (137.846)	-68.56 (72.548)	368.63 (338.158)	29.33 (272.487)		
Median	222.00	-44.50	260.50	-11.00		
Min, Max	126.0, 953.0	-307.5, 20.5	161.0, 1260.0	-345.0, 955.0		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Source: ADaM.ADSL, ADaM.ADLB
Run Date: 2024-04-26T15:50:26
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.5

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	32	31	14	13		
Mean (SD)	217.78 (58.707)	-75.05 (74.790)	333.79 (273.631)	-3.35 (68.528)		
Median	216.50	-58.00	262.50	-10.00		
Min, Max	121.0, 342.0	-301.5, 9.5	182.0, 1260.0	-83.0, 193.0		
Week 4						
n	32	31	14	13		
Mean (SD)	260.72 (129.417)	-47.73 (71.093)	343.36 (268.398)	7.31 (39.918)		
Median	256.50	-38.00	275.00	14.00		
Min, Max	108.0, 847.0	-224.5, 114.0	194.0, 1260.0	-82.5, 76.5		
Week 6						
n	31	30	15	14		
Mean (SD)	250.90 (76.248)	-49.68 (76.215)	378.80 (292.254)	52.14 (169.914)		
Median	250.00	-47.50	265.00	6.00		
Min, Max	124.0, 529.0	-238.5, 78.0	176.0, 1260.0	-98.5, 603.0		

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	33	32	15	14		
Mean (SD)	270.79 (84.154)	-24.47 (58.634)	370.93 (272.675)	43.71 (125.304)		
Median	257.00	-32.75	278.00	15.50		
Min, Max	145.0, 475.0	-160.0, 123.0	190.0, 1260.0	-113.5, 432.0		
Week 10						
n	31	30	15	14		
Mean (SD)	254.19 (53.069)	-35.57 (77.364)	345.27 (265.061)	16.21 (81.791)		
Median	251.00	-19.50	258.00	0.50		
Min, Max	158.0, 399.0	-213.0, 82.5	193.0, 1260.0	-98.5, 191.5		
Week 12						
n	30	29	15	14		
Mean (SD)	253.00 (55.645)	-36.50 (80.409)	335.00 (261.995)	5.21 (46.907)		
Median	256.50	-16.50	251.00	3.50		
Min, Max	142.0, 355.0	-230.5, 99.5	199.0, 1260.0	-84.5, 80.0		

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	32	31	13	12		
Mean (SD)	281.44 (129.220)	-10.65 (148.133)	269.00 (180.193)	-27.13 (40.943)		
Median	268.00	1.00	223.00	-8.75		
Min, Max	129.0, 882.0	-259.5, 627.5	145.0, 854.0	-102.0, 27.5		
Week 14						
n	31	30	14	13		
Mean (SD)	261.68 (86.583)	-30.00 (95.184)	254.07 (82.996)	-20.69 (45.399)		
Median	249.00	-21.50	227.00	-5.00		
Min, Max	135.0, 573.0	-243.5, 205.5	165.0, 501.0	-135.5, 22.5		
Week 15						
n	30	29	13	12		
Mean (SD)	256.57 (67.963)	-36.33 (86.377)	232.62 (51.016)	-32.88 (39.712)		
Median	254.50	-26.00	234.00	-20.25		
Min, Max	142.0, 411.0	-243.5, 209.5	154.0, 361.0	-122.5, 11.5		

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	32	31	14	13		
Mean (SD)	270.22 (76.611)	-20.61 (87.518)	259.00 (65.255)	-1.31 (86.543)		
Median	268.50	-23.00	249.50	-10.50		
Min, Max	159.0, 447.0	-239.5, 210.5	156.0, 439.0	-97.5, 252.0		
Week 18						
n	32	31	13	12		
Mean (SD)	254.84 (62.896)	-35.26 (81.744)	256.85 (55.646)	-0.63 (42.252)		
Median	255.50	-23.00	282.00	-4.25		
Min, Max	159.0, 419.0	-222.5, 100.5	160.0, 352.0	-73.5, 61.0		
Week 20						
n	31	30	14	13		
Mean (SD)	273.90 (87.415)	-23.13 (94.264)	283.43 (86.998)	22.69 (72.120)		
Median	261.00	-12.25	259.50	3.00		
Min, Max	163.0, 630.0	-243.5, 235.5	179.0, 535.0	-66.5, 229.0		

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	32	31	14	13		
Mean (SD)	252.19 (67.343)	-46.03 (75.861)	286.29 (59.817)	27.00 (46.131)		
Median	254.00	-21.00	289.00	17.00		
Min, Max	108.0, 405.0	-224.5, 78.5	180.0, 416.0	-67.5, 110.0		
Week 24						
n	31	30	14	13		
Mean (SD)	276.39 (92.581)	-22.40 (84.100)	300.64 (75.954)	41.15 (59.489)		
Median	269.00	-18.50	305.00	51.00		
Min, Max	139.0, 573.0	-201.5, 130.5	188.0, 442.0	-59.5, 136.0		
Week 28						
n	29	29	14	13	43	42
Mean (SD)	267.10 (90.337)	-24.67 (106.523)	296.71 (70.819)	38.00 (54.843)	276.74 (84.794)	-5.27 (97.414)
Median	257.00	-28.00	294.50	27.50	268.00	-7.25
Min, Max	156.0, 632.0	-208.0, 370.5	195.0, 433.0	-65.5, 127.0	156.0, 632.0	-208.0, 370.5

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	29	28	13	12	42	40
Mean (SD)	267.45 (77.358)	-32.16 (84.226)	302.38 (75.002)	39.88 (64.814)	278.26 (77.462)	-10.55 (84.934)
Median	250.00	-13.25	279.00	29.75	261.00	-1.50
Min, Max	161.0, 484.0	-255.5, 101.0	216.0, 421.0	-93.5, 153.5	161.0, 484.0	-255.5, 153.5
Week 36						
n	28	27	13	12	41	39
Mean (SD)	269.39 (114.351)	-30.81 (125.685)	293.38 (78.982)	32.63 (60.295)	277.00 (104.046)	-11.29 (112.874)
Median	251.50	-28.50	277.00	15.00	256.00	-10.00
Min, Max	168.0, 804.0	-229.5, 470.0	217.0, 478.0	-62.5, 172.0	168.0, 804.0	-229.5, 470.0
Week 40						
n	27	26	13	12	40	38
Mean (SD)	285.63 (86.659)	-16.77 (94.157)	298.15 (65.088)	35.38 (49.679)	289.70 (79.657)	-0.30 (85.600)
Median	269.00	-9.25	302.00	42.75	273.00	-7.00
Min, Max	170.0, 623.0	-230.5, 153.0	200.0, 394.0	-34.5, 120.0	170.0, 623.0	-230.5, 153.0

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Post-hoc Analysen Studie ALPHA

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	26	25	13	12	39	37
Mean (SD)	258.27 (52.397)	-39.76 (60.705)	285.54 (49.682)	24.13 (37.719)	267.36 (52.491)	-19.04 (61.730)
Median	266.50	-31.00	291.00	32.75	269.00	-19.00
Min, Max	181.0, 347.0	-210.5, 93.0	197.0, 373.0	-62.5, 68.0	181.0, 373.0	-210.5, 93.0
Week 48						
n	23	23	12	11	35	34
Mean (SD)	281.96 (69.989)	-10.54 (84.761)	290.58 (66.146)	33.77 (41.442)	284.91 (67.841)	3.79 (75.848)
Median	279.00	-12.50	276.50	31.00	277.00	-6.25
Min, Max	180.0, 455.0	-198.5, 230.0	206.0, 413.0	-17.0, 125.5	180.0, 455.0	-198.5, 230.0
Week 52						
n	21	21	11	10	32	31
Mean (SD)	267.81 (59.776)	-23.12 (57.108)	285.27 (73.410)	26.80 (62.069)	273.81 (64.146)	-7.02 (62.391)
Median	258.00	-23.00	264.00	33.75	258.50	-10.00
Min, Max	184.0, 360.0	-170.5, 82.5	198.0, 455.0	-92.5, 149.0	184.0, 455.0	-170.5, 149.0

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	22	22	11	10	33	32
Mean (SD)	291.91 (98.325)	0.75 (105.123)	284.91 (69.031)	25.10 (65.511)	289.58 (88.572)	8.36 (94.146)
Median	281.50	-5.25	256.00	27.25	272.00	0.25
Min, Max	161.0, 635.0	-233.5, 339.0	204.0, 389.0	-87.5, 149.0	161.0, 635.0	-233.5, 339.0
Week 60						
n	19	19	10	9	29	28
Mean (SD)	249.74 (42.960)	-38.21 (64.487)	282.50 (56.301)	20.78 (48.367)	261.03 (49.563)	-19.25 (65.212)
Median	252.00	-23.00	266.00	33.00	252.00	-8.25
Min, Max	168.0, 309.0	-199.5, 60.5	213.0, 376.0	-67.5, 98.0	168.0, 376.0	-199.5, 98.0
Week 64						
n	15	15	8	7	23	22
Mean (SD)	274.73 (54.018)	-24.93 (76.857)	262.25 (67.911)	15.50 (40.206)	270.39 (57.976)	-12.07 (69.075)
Median	261.00	-16.00	227.50	34.00	254.00	-14.50
Min, Max	190.0, 366.0	-227.5, 84.0	209.0, 390.0	-43.5, 57.5	190.0, 390.0	-227.5, 84.0

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	12	12	7	7	19	19
Mean (SD)	262.17 (47.838)	-23.75 (85.417)	253.43 (49.527)	24.93 (48.225)	258.95 (47.275)	-5.82 (76.262)
Median	277.50	10.50	259.00	23.50	272.00	22.00
Min, Max	137.0, 303.0	-192.0, 64.0	198.0, 343.0	-69.5, 85.0	137.0, 343.0	-192.0, 85.0
Week 72						
n	11	11	6	6	17	17
Mean (SD)	265.18 (62.323)	-23.32 (94.262)	248.83 (36.086)	30.17 (49.164)	259.41 (53.846)	-4.44 (83.683)
Median	268.00	-3.50	249.00	25.00	257.00	16.00
Min, Max	184.0, 403.0	-206.5, 78.0	196.0, 307.0	-22.5, 120.0	184.0, 403.0	-206.5, 120.0
Week 80						
n	10	10	5	5	15	15
Mean (SD)	264.50 (48.539)	-31.45 (76.475)	214.80 (36.915)	-0.50 (40.884)	247.93 (49.921)	-21.13 (66.823)
Median	273.00	-21.75	239.00	-2.00	242.00	-6.00
Min, Max	188.0, 329.0	-162.5, 61.5	171.0, 244.0	-52.0, 52.0	171.0, 329.0	-162.5, 61.5

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.5

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	4	4	5	5	9	9
Mean (SD)	290.75 (45.043)	27.88 (32.253)	242.20 (26.109)	24.40 (30.613)	263.78 (41.910)	25.94 (29.360)
Median	285.50	37.00	246.00	32.00	263.00	34.50
Min, Max	242.0, 350.0	-18.5, 56.0	212.0, 271.0	-16.5, 59.0	212.0, 350.0	-18.5, 59.0
Week 96						
n	2	2	4	4	6	6
Mean (SD)	239.00 (15.556)	-38.25 (39.244)	234.00 (46.583)	6.75 (63.225)	235.67 (36.838)	-8.25 (56.978)
Median	239.00	-38.25	228.50	1.25	229.00	-25.50
Min, Max	228.0, 250.0	-66.0, -10.5	183.0, 296.0	-52.5, 77.0	183.0, 296.0	-66.0, 77.0
Week 104						
n	0	0	1	1	1	1
Mean (SD)			221.00 (NA)	34.00 (NA)	221.00 (NA)	34.00 (NA)
Median			221.00	34.00	221.00	34.00
Min, Max			221.0, 221.0	34.0, 34.0	221.0, 221.0	34.0, 34.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.6

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		14		49	
Mean (SD)	334.05 (137.793)		279.46 (77.952)		318.46 (125.362)	
Median	307.00		257.25		270.50	
Min, Max	165.5, 809.0		139.0, 404.0		139.0, 809.0	
Week 1						
n	31	31	14	13		
Mean (SD)	258.94 (84.558)	-81.91 (78.585)	349.79 (275.356)	-3.08 (60.960)		
Median	241.00	-58.00	266.50	-11.00		
Min, Max	151.0, 532.0	-295.5, 9.5	128.0, 1260.0	-89.0, 155.0		
Week 2						
n	35	35	15	14		
Mean (SD)	249.40 (83.758)	-84.65 (86.418)	387.60 (349.163)	45.82 (265.821)		
Median	240.00	-60.00	235.00	-9.00		
Min, Max	126.0, 515.0	-307.5, 53.0	144.0, 1260.0	-155.0, 955.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.6

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	33	33	14	13		
Mean (SD)	248.36 (82.221)	-88.85 (95.205)	331.79 (281.586)	-22.46 (85.860)		
Median	233.00	-60.00	250.50	-10.50		
Min, Max	121.0, 540.0	-398.5, 24.0	123.0, 1260.0	-185.0, 193.0		
Week 4						
n	34	34	12	11		
Mean (SD)	266.21 (94.934)	-67.80 (84.059)	356.92 (295.349)	4.73 (35.269)		
Median	260.00	-55.25	281.00	-5.00		
Min, Max	108.0, 551.0	-259.5, 114.0	123.0, 1260.0	-29.0, 76.5		
Week 6						
n	33	33	15	14		
Mean (SD)	280.48 (136.126)	-56.49 (70.129)	385.20 (298.605)	43.25 (176.898)		
Median	259.00	-49.00	258.00	16.75		
Min, Max	124.0, 794.0	-238.5, 59.5	130.0, 1260.0	-167.0, 603.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.8.6

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	33	33	14	13		
Mean (SD)	299.33 (114.255)	-35.75 (96.527)	396.43 (282.498)	57.65 (119.922)		
Median	288.00	-26.50	347.00	28.00		
Min, Max	145.0, 629.0	-337.5, 255.5	122.0, 1260.0	-22.0, 432.0		
Week 10						
n	30	30	15	14		
Mean (SD)	272.70 (84.665)	-45.11 (74.349)	347.53 (270.999)	2.89 (91.155)		
Median	266.00	-22.00	256.00	2.25		
Min, Max	148.0, 589.0	-220.0, 45.0	155.0, 1260.0	-137.0, 191.5		
Week 12						
n	32	32	15	14		
Mean (SD)	268.00 (81.587)	-62.23 (98.230)	348.40 (264.723)	3.82 (34.743)		
Median	250.50	-28.75	304.00	-1.00		
Min, Max	135.0, 519.0	-379.5, 53.0	128.0, 1260.0	-54.5, 64.5		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.6

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	32	32	14	13		
Mean (SD)	285.44 (143.927)	-49.28 (163.689)	265.86 (176.970)	-52.12 (74.554)		
Median	252.50	-26.00	230.50	-17.00		
Min, Max	129.0, 882.0	-496.5, 627.5	122.0, 854.0	-196.0, 27.5		
Week 14						
n	33	33	13	12		
Mean (SD)	269.55 (85.105)	-61.51 (116.649)	244.92 (91.366)	-44.04 (69.152)		
Median	260.00	-25.00	223.00	-9.50		
Min, Max	153.0, 587.0	-442.5, 205.5	118.0, 501.0	-189.0, 22.5		
Week 15						
n	30	30	12	11		
Mean (SD)	264.20 (91.074)	-73.67 (105.355)	227.00 (61.921)	-49.36 (71.314)		
Median	252.50	-40.25	221.00	-19.00		
Min, Max	134.0, 627.0	-486.5, 36.0	123.0, 361.0	-168.0, 29.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.6

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	33	33	14	13		
Mean (SD)	297.79 (110.354)	-33.27 (112.977)	242.93 (67.086)	-35.58 (68.620)		
Median	280.00	-21.50	242.00	-18.00		
Min, Max	144.0, 639.0	-258.5, 332.0	121.0, 390.0	-182.0, 50.0		
Week 18						
n	33	33	13	12		
Mean (SD)	273.58 (87.553)	-57.48 (90.374)	274.08 (78.432)	-0.33 (63.076)		
Median	262.00	-33.00	282.00	2.75		
Min, Max	163.0, 568.0	-297.5, 61.5	141.0, 461.0	-111.0, 93.5		
Week 20						
n	30	30	14	13		
Mean (SD)	290.23 (132.522)	-48.53 (90.150)	262.86 (57.718)	-16.42 (59.932)		
Median	260.50	-38.50	263.00	-12.00		
Min, Max	157.0, 725.0	-243.5, 235.5	137.0, 348.0	-127.0, 84.5		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.6

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	33	33	14	13		
Mean (SD)	275.09 (135.159)	-55.96 (87.229)	259.07 (48.732)	-19.27 (82.580)		
Median	255.00	-47.00	257.00	-1.50		
Min, Max	139.0, 906.0	-307.0, 171.5	169.0, 332.0	-180.0, 83.5		
Week 24						
n	31	31	13	12		
Mean (SD)	281.06 (101.473)	-55.88 (121.684)	283.23 (49.014)	-6.13 (91.292)		
Median	262.00	-37.50	264.00	25.75		
Min, Max	130.0, 573.0	-491.5, 142.0	209.0, 366.0	-125.0, 135.5		
Week 28						
n	33	33	13	12	46	45
Mean (SD)	284.48 (107.613)	-46.57 (122.854)	274.23 (56.679)	-14.71 (87.405)	281.59 (95.465)	-38.07 (114.410)
Median	267.00	-28.00	298.00	-3.00	270.50	-24.50
Min, Max	134.0, 632.0	-467.5, 370.5	173.0, 342.0	-161.0, 111.5	134.0, 632.0	-467.5, 370.5

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.6

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	29	29	12	11	41	40
Mean (SD)	281.97 (96.581)	-57.10 (101.946)	292.25 (83.702)	-2.45 (120.773)	284.98 (92.079)	-42.08 (108.685)
Median	250.00	-22.50	263.00	22.00	256.00	-12.25
Min, Max	150.0, 512.0	-331.5, 79.5	192.0, 421.0	-167.0, 153.5	150.0, 512.0	-331.5, 153.5
Week 36						
n	29	29	10	9	39	38
Mean (SD)	276.34 (122.235)	-26.13 (123.255)	281.00 (62.823)	0.83 (45.541)	277.54 (109.309)	-19.75 (109.908)
Median	248.00	-43.50	279.50	-5.50	256.00	-30.00
Min, Max	131.0, 804.0	-229.5, 470.0	172.0, 354.0	-52.0, 107.0	131.0, 804.0	-229.5, 470.0
Week 40						
n	29	29	11	10	40	39
Mean (SD)	277.41 (70.592)	-43.36 (113.904)	278.27 (57.672)	-22.50 (90.646)	277.65 (66.563)	-38.01 (107.662)
Median	277.00	-22.00	267.00	-26.25	271.00	-22.00
Min, Max	170.0, 516.0	-395.5, 209.0	170.0, 360.0	-164.0, 120.0	170.0, 516.0	-395.5, 209.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:28

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.6

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	27	27	11	10	38	37
Mean (SD)	262.56 (59.831)	-57.44 (96.375)	259.00 (48.440)	-28.30 (83.307)	261.53 (56.146)	-49.56 (92.818)
Median	269.00	-32.50	252.00	-13.00	266.50	-31.00
Min, Max	149.0, 394.0	-443.5, 64.0	164.0, 328.0	-180.0, 81.0	149.0, 394.0	-443.5, 81.0
Week 48						
n	21	21	12	11	33	32
Mean (SD)	284.33 (74.472)	-22.62 (89.002)	280.00 (72.043)	-15.55 (79.758)	282.76 (72.491)	-20.19 (84.701)
Median	281.00	-17.50	276.50	7.50	279.00	-16.50
Min, Max	157.0, 455.0	-198.5, 230.0	170.0, 413.0	-164.0, 125.5	157.0, 455.0	-198.5, 230.0
Week 52						
n	22	22	12	11	34	33
Mean (SD)	278.91 (62.182)	-47.48 (105.914)	270.58 (59.422)	-27.00 (91.194)	275.97 (60.447)	-40.65 (100.283)
Median	290.50	-25.75	266.00	-27.00	280.50	-27.00
Min, Max	155.0, 387.0	-436.5, 67.0	175.0, 368.0	-189.0, 100.0	155.0, 387.0	-436.5, 100.0

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	22	22	11	10	33	32
Mean (SD)	297.36 (78.893)	-29.02 (107.508)	314.09 (134.402)	14.50 (142.401)	302.94 (98.963)	-15.42 (118.899)
Median	285.00	-13.50	255.00	-2.75	283.00	-13.50
Min, Max	153.0, 439.0	-329.5, 168.5	186.0, 673.0	-149.0, 339.0	153.0, 673.0	-329.5, 339.0
Week 60						
n	20	20	9	8	29	28
Mean (SD)	269.15 (72.979)	-37.48 (85.892)	289.11 (84.945)	-11.38 (75.877)	275.34 (75.921)	-30.02 (82.634)
Median	270.50	-41.75	253.00	-14.00	261.00	-37.00
Min, Max	161.0, 444.0	-199.5, 204.0	166.0, 444.0	-151.0, 110.0	161.0, 444.0	-199.5, 204.0
Week 64						
n	18	18	9	8	27	26
Mean (SD)	276.44 (60.002)	-38.39 (71.057)	288.67 (87.350)	-13.63 (72.228)	280.52 (68.820)	-30.77 (70.922)
Median	287.00	-20.25	262.00	-27.25	275.00	-22.00
Min, Max	151.0, 354.0	-227.5, 66.5	167.0, 421.0	-131.5, 87.0	151.0, 421.0	-227.5, 87.0

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	14	14	7	7	21	21
Mean (SD)	277.21 (62.385)	-17.79 (61.806)	245.14 (58.769)	-38.14 (72.489)	266.52 (61.692)	-24.57 (64.468)
Median	277.50	-5.75	236.00	-40.00	275.00	-14.00
Min, Max	143.0, 412.0	-179.5, 64.0	162.0, 343.0	-131.5, 55.5	143.0, 412.0	-179.5, 64.0
Week 72						
n	11	11	6	6	17	17
Mean (SD)	263.55 (74.377)	-24.91 (87.527)	240.67 (31.123)	-41.92 (65.236)	255.47 (62.347)	-30.91 (78.665)
Median	269.00	-18.50	249.00	-21.25	257.00	-20.00
Min, Max	142.0, 403.0	-206.5, 78.0	182.0, 266.0	-138.0, 34.0	142.0, 403.0	-206.5, 78.0
Week 80						
n	9	9	5	5	14	14
Mean (SD)	273.11 (58.360)	-28.00 (67.603)	271.40 (76.035)	-20.60 (34.856)	272.50 (62.254)	-25.36 (56.567)
Median	279.00	-12.50	244.00	-15.00	276.50	-15.00
Min, Max	153.0, 355.0	-162.5, 39.0	187.0, 389.0	-72.5, 25.0	153.0, 389.0	-162.5, 39.0

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Post-hoc Analysen Studie ALPHA

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Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	5	5	11	11
Mean (SD)	269.33 (62.516)	-4.67 (46.315)	247.60 (30.163)	-10.70 (51.165)	259.45 (49.466)	-7.41 (46.147)
Median	271.00	10.25	263.00	3.50	263.00	9.00
Min, Max	176.0, 362.0	-91.0, 39.5	211.0, 274.0	-93.5, 44.0	176.0, 362.0	-93.5, 44.0
Week 96						
n	4	4	5	5	9	9
Mean (SD)	252.25 (74.888)	-27.50 (52.110)	245.20 (71.880)	-13.10 (51.705)	248.33 (68.558)	-19.50 (49.118)
Median	248.00	-5.50	227.00	-25.50	246.00	-24.00
Min, Max	165.0, 348.0	-105.0, 6.0	178.0, 342.0	-52.5, 77.0	165.0, 348.0	-105.0, 77.0
Week 104						
n	0	0	1	1	1	1
Mean (SD)			291.00 (NA)	-76.50 (NA)	291.00 (NA)	-76.50 (NA)
Median			291.00	-76.50	291.00	-76.50
Min, Max			291.0, 291.0	-76.5, -76.5	291.0, 291.0	-76.5, -76.5

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		11		32	
Mean (SD)	253.90 (74.283)		279.68 (96.459)		262.77 (81.951)	
Median	237.50		258.50		240.00	
Min, Max	140.0, 476.0		180.0, 506.0		140.0, 506.0	
Week 1						
n	20	19	11	11		
Mean (SD)	228.35 (73.707)	-39.32 (54.114)	239.36 (40.847)	-40.32 (103.529)		
Median	212.50	-42.00	243.00	-17.50		
Min, Max	137.0, 457.0	-169.0, 87.0	174.0, 315.0	-332.0, 56.0		
Week 2						
n	22	21	11	11		
Mean (SD)	246.05 (166.815)	-41.52 (57.217)	248.82 (50.221)	-30.86 (109.606)		
Median	217.50	-41.50	261.00	0.50		
Min, Max	117.0, 953.0	-199.0, 66.5	161.0, 321.0	-345.0, 76.0		

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Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	21	20	10	10		
Mean (SD)	205.10 (56.272)	-56.48 (57.819)	262.80 (71.027)	5.75 (68.271)		
Median	201.00	-47.00	262.50	1.25		
Min, Max	122.0, 342.0	-263.0, 9.5	182.0, 421.0	-77.5, 176.0		
Week 4						
n	22	21	10	10		
Mean (SD)	261.91 (146.486)	-19.86 (60.246)	256.90 (47.832)	-0.15 (35.982)		
Median	230.00	-11.00	263.50	13.75		
Min, Max	138.0, 847.0	-209.0, 98.5	194.0, 328.0	-82.5, 32.0		
Week 6						
n	21	20	10	10		
Mean (SD)	261.62 (80.431)	-6.68 (68.007)	251.20 (44.504)	-5.85 (60.021)		
Median	250.00	-13.00	261.50	-1.50		
Min, Max	146.0, 529.0	-201.0, 142.0	176.0, 312.0	-98.5, 125.0		

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Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	21	20	10	10		
Mean (SD)	259.33 (82.736)	-2.83 (63.239)	247.60 (32.681)	-9.45 (47.622)		
Median	242.00	-14.75	255.00	1.25		
Min, Max	146.0, 475.0	-160.0, 123.0	190.0, 291.0	-113.5, 64.0		
Week 10						
n	21	20	10	10		
Mean (SD)	249.29 (54.160)	-7.58 (62.068)	249.60 (37.447)	-7.45 (44.220)		
Median	246.00	-10.00	252.00	9.00		
Min, Max	158.0, 399.0	-213.0, 82.5	193.0, 311.0	-98.5, 44.0		
Week 12						
n	20	19	10	10		
Mean (SD)	261.25 (51.255)	10.42 (63.253)	252.60 (56.912)	-4.45 (45.783)		
Median	269.00	11.50	248.00	5.75		
Min, Max	171.0, 355.0	-200.0, 99.5	188.0, 386.0	-84.5, 80.0		

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Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	21	20	9	9		
Mean (SD)	251.57 (64.161)	-0.48 (68.717)	196.67 (31.205)	-49.33 (39.052)		
Median	251.00	2.00	203.00	-42.00		
Min, Max	146.0, 407.0	-223.0, 133.5	145.0, 232.0	-103.0, 1.0		
Week 14						
n	20	19	10	10		
Mean (SD)	279.10 (111.126)	28.89 (77.910)	220.10 (49.870)	-36.95 (51.210)		
Median	260.50	1.00	220.50	-20.00		
Min, Max	135.0, 573.0	-46.0, 214.5	158.0, 322.0	-135.5, 16.0		
Week 15						
n	21	20	10	10		
Mean (SD)	274.90 (98.568)	20.95 (97.999)	210.70 (40.033)	-46.35 (36.778)		
Median	249.00	5.50	214.50	-29.50		
Min, Max	142.0, 521.0	-174.0, 283.5	154.0, 279.0	-122.5, -13.0		

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Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	21	20	10	10		
Mean (SD)	265.05 (95.055)	12.95 (88.638)	250.70 (79.485)	-6.35 (102.150)		
Median	226.00	-3.00	247.50	-8.75		
Min, Max	159.0, 522.0	-146.0, 243.5	156.0, 439.0	-108.0, 252.0		
Week 18						
n	21	20	10	10		
Mean (SD)	256.48 (66.845)	5.85 (68.071)	242.30 (60.019)	-14.75 (50.493)		
Median	260.00	4.00	238.50	-19.00		
Min, Max	159.0, 392.0	-191.0, 113.5	160.0, 352.0	-94.0, 67.5		
Week 20						
n	19	18	10	10		
Mean (SD)	269.00 (56.827)	5.86 (56.922)	270.50 (106.627)	13.45 (88.518)		
Median	268.00	18.25	236.50	2.50		
Min, Max	171.0, 400.0	-159.0, 89.5	179.0, 535.0	-109.0, 229.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Source: ADaM.ADSL, ADaM.ADLB
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.6

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	21	20	10	10		
Mean (SD)	256.33 (71.658)	-6.75 (60.032)	274.70 (72.771)	17.65 (61.719)		
Median	260.00	-6.50	275.50	17.00		
Min, Max	108.0, 405.0	-192.0, 78.5	180.0, 416.0	-95.0, 110.0		
Week 24						
n	21	20	10	10		
Mean (SD)	269.48 (78.061)	8.65 (61.123)	277.30 (91.547)	20.25 (68.130)		
Median	269.00	8.00	267.50	31.00		
Min, Max	139.0, 468.0	-175.0, 116.0	183.0, 442.0	-88.0, 136.0		
Week 28						
n	18	18	10	10	28	28
Mean (SD)	249.11 (58.049)	0.08 (81.199)	285.20 (82.526)	28.15 (77.276)	262.00 (68.571)	10.11 (79.557)
Median	249.50	12.75	268.50	21.25	254.00	16.75
Min, Max	173.0, 341.0	-208.0, 114.0	193.0, 433.0	-101.0, 143.5	173.0, 433.0	-208.0, 143.5

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.6

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	19	18	10	10	29	28
Mean (SD)	272.37 (73.618)	6.67 (54.668)	250.90 (70.040)	-6.15 (59.221)	264.97 (71.894)	2.09 (55.586)
Median	270.00	20.50	235.00	-4.00	249.00	8.50
Min, Max	173.0, 484.0	-149.0, 101.0	154.0, 408.0	-103.0, 74.0	154.0, 484.0	-149.0, 101.0
Week 36						
n	18	17	10	10	28	27
Mean (SD)	259.22 (50.697)	-5.15 (59.970)	282.90 (99.119)	25.85 (75.350)	267.68 (70.898)	6.33 (66.417)
Median	267.00	6.00	242.00	30.00	261.50	6.00
Min, Max	185.0, 384.0	-172.0, 62.0	166.0, 478.0	-62.5, 172.0	166.0, 478.0	-172.0, 172.0
Week 40						
n	17	16	10	10	27	26
Mean (SD)	293.35 (103.642)	28.78 (62.565)	264.20 (81.218)	7.15 (56.638)	282.56 (95.391)	20.46 (60.155)
Median	259.00	11.50	231.00	7.00	257.00	11.50
Min, Max	201.0, 623.0	-56.0, 153.0	177.0, 394.0	-117.0, 88.0	177.0, 623.0	-117.0, 153.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.6

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	16	15	9	9	25	24
Mean (SD)	261.38 (53.869)	-5.07 (57.040)	270.78 (70.784)	5.89 (64.229)	264.76 (59.203)	-0.96 (58.692)
Median	271.50	-12.50	291.00	39.00	278.00	-10.00
Min, Max	181.0, 347.0	-129.0, 93.0	176.0, 373.0	-118.0, 68.0	176.0, 373.0	-129.0, 93.0
Week 48						
n	15	15	8	8	23	23
Mean (SD)	281.93 (72.316)	14.00 (60.365)	263.63 (66.436)	7.38 (56.643)	275.57 (69.367)	11.70 (57.880)
Median	250.00	9.50	237.50	10.75	249.00	9.50
Min, Max	182.0, 420.0	-118.0, 126.5	193.0, 347.0	-101.0, 76.5	182.0, 420.0	-118.0, 126.5
Week 52						
n	13	13	7	7	20	20
Mean (SD)	242.15 (54.601)	-16.23 (53.274)	266.29 (85.646)	10.36 (78.990)	250.60 (65.869)	-6.93 (62.707)
Median	235.00	-16.00	241.00	18.00	238.00	-7.00
Min, Max	184.0, 330.0	-146.0, 82.5	198.0, 455.0	-92.5, 149.0	184.0, 455.0	-146.0, 149.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.6

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	14	14	7	7	21	21
Mean (SD)	295.14 (119.301)	31.07 (120.086)	276.86 (59.269)	20.93 (56.807)	289.05 (101.898)	27.69 (101.811)
Median	276.00	0.75	269.00	41.00	272.00	13.50
Min, Max	161.0, 635.0	-154.0, 339.0	204.0, 378.0	-87.5, 72.0	161.0, 635.0	-154.0, 339.0
Week 60						
n	11	11	7	7	18	18
Mean (SD)	255.73 (50.488)	-2.32 (67.440)	259.86 (61.407)	3.93 (74.906)	257.33 (53.241)	0.11 (68.304)
Median	252.00	1.50	238.00	33.00	250.00	13.75
Min, Max	181.0, 313.0	-167.0, 75.5	175.0, 347.0	-119.0, 98.0	175.0, 347.0	-167.0, 98.0
Week 64						
n	10	10	5	5	15	15
Mean (SD)	278.30 (55.394)	21.75 (65.578)	218.20 (17.641)	-7.60 (59.011)	258.27 (54.051)	11.97 (62.966)
Median	283.50	53.50	221.00	-8.00	242.00	34.00
Min, Max	190.0, 366.0	-110.0, 84.0	193.0, 237.0	-101.0, 51.0	190.0, 366.0	-110.0, 84.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.6

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	9	9	5	5	14	14
Mean (SD)	259.67 (58.314)	5.56 (91.213)	229.20 (42.681)	3.40 (78.446)	248.79 (53.690)	4.79 (83.753)
Median	284.00	31.00	233.00	22.00	263.00	26.50
Min, Max	137.0, 333.0	-192.0, 99.5	172.0, 272.0	-122.0, 85.0	137.0, 333.0	-192.0, 99.5
Week 72						
n	8	8	5	5	13	13
Mean (SD)	247.50 (41.078)	-8.69 (79.641)	230.40 (55.886)	4.60 (91.454)	240.92 (45.830)	-3.58 (80.828)
Median	247.00	17.50	229.00	16.00	229.00	16.00
Min, Max	191.0, 312.0	-193.0, 69.5	162.0, 307.0	-132.0, 120.0	162.0, 312.0	-193.0, 120.0
Week 80						
n	8	8	3	3	11	11
Mean (SD)	281.50 (86.530)	25.31 (111.521)	196.00 (37.403)	-0.67 (52.013)	258.18 (84.356)	18.23 (96.923)
Median	269.00	18.00	178.00	-2.00	234.00	-2.00
Min, Max	188.0, 449.0	-147.0, 215.5	171.0, 239.0	-52.0, 52.0	171.0, 449.0	-147.0, 215.5

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:28

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.6

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	5	5	2	2	7	7
Mean (SD)	349.00 (111.452)	107.50 (130.729)	229.00 (24.042)	45.50 (19.092)	314.71 (108.655)	89.79 (111.218)
Median	350.00	56.00	229.00	45.50	277.00	56.00
Min, Max	239.0, 529.0	1.5, 329.0	212.0, 246.0	32.0, 59.0	212.0, 529.0	1.5, 329.0
Week 96						
n	3	3	1	1	4	4
Mean (SD)	247.67 (41.187)	-7.33 (64.355)	230.00 (NA)	43.00 (NA)	243.25 (34.769)	5.25 (58.262)
Median	228.00	-17.50	230.00	43.00	229.00	12.75
Min, Max	220.0, 295.0	-66.0, 61.5	230.0, 230.0	43.0, 43.0	220.0, 295.0	-66.0, 61.5
Week 104						
n	1	1	1	1	2	2
Mean (SD)	193.00 (NA)	-44.50 (NA)	221.00 (NA)	34.00 (NA)	207.00 (19.799)	-5.25 (55.508)
Median	193.00	-44.50	221.00	34.00	207.00	-5.25
Min, Max	193.0, 193.0	-44.5, -44.5	221.0, 221.0	34.0, 34.0	193.0, 221.0	-44.5, 34.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:28

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	20		12		32	
Mean (SD)	277.82 (120.563)		300.79 (84.061)		286.43 (107.443)	
Median	250.00		280.75		254.50	
Min, Max	165.5, 734.5		219.0, 506.0		165.5, 734.5	
Week 1						
n	18	17	11	11		
Mean (SD)	238.50 (88.486)	-49.81 (70.145)	280.36 (81.235)	-26.36 (121.030)		
Median	221.00	-35.00	252.00	-11.00		
Min, Max	137.0, 457.0	-295.5, 33.0	174.0, 404.0	-332.0, 155.0		
Week 2						
n	21	20	12	12		
Mean (SD)	259.29 (177.255)	-53.22 (69.757)	335.33 (278.786)	34.54 (308.306)		
Median	222.00	-36.50	261.50	-14.00		
Min, Max	126.0, 953.0	-288.5, 23.0	161.0, 1195.0	-345.0, 955.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	20	19	10	10		
Mean (SD)	223.65 (69.039)	-64.16 (91.340)	294.70 (94.503)	7.90 (114.363)		
Median	230.50	-40.00	267.50	-8.25		
Min, Max	121.0, 344.0	-398.5, 24.0	149.0, 433.0	-185.0, 193.0		
Week 4						
n	21	20	8	8		
Mean (SD)	265.38 (160.606)	-41.52 (74.863)	293.75 (56.928)	23.50 (35.872)		
Median	212.00	-22.75	283.00	24.50		
Min, Max	108.0, 847.0	-259.5, 45.5	214.0, 389.0	-40.0, 76.5		
Week 6						
n	20	19	11	11		
Mean (SD)	280.15 (149.140)	-14.12 (51.893)	321.64 (184.677)	39.50 (200.666)		
Median	254.50	-25.50	264.00	11.00		
Min, Max	124.0, 794.0	-116.5, 78.0	167.0, 843.0	-167.0, 603.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:31

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	20	19	10	10		
Mean (SD)	282.55 (122.141)	-3.10 (110.316)	331.10 (130.158)	57.95 (137.762)		
Median	246.00	-15.33	280.00	13.75		
Min, Max	145.0, 629.0	-337.5, 255.5	225.0, 672.0	-31.0, 432.0		
Week 10						
n	19	18	11	11		
Mean (SD)	255.42 (61.551)	6.26 (38.829)	295.09 (86.626)	12.95 (102.043)		
Median	231.00	-9.92	260.00	8.50		
Min, Max	148.0, 399.0	-66.5, 82.5	197.0, 459.0	-137.0, 191.5		
Week 12						
n	18	17	11	11		
Mean (SD)	246.94 (65.995)	-25.46 (101.422)	300.27 (61.716)	18.14 (41.954)		
Median	244.50	-10.00	304.00	15.00		
Min, Max	135.0, 355.0	-379.5, 99.5	223.0, 394.0	-49.0, 80.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:31

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	18	17	11	11		
Mean (SD)	278.50 (166.158)	4.92 (204.913)	214.55 (30.778)	-67.59 (71.640)		
Median	238.50	-10.50	219.00	-64.50		
Min, Max	129.0, 882.0	-496.5, 627.5	138.0, 247.0	-196.0, 7.0		
Week 14						
n	18	17	10	10		
Mean (SD)	264.67 (92.012)	-7.05 (137.827)	224.60 (39.030)	-52.35 (73.780)		
Median	255.50	9.00	220.00	-18.75		
Min, Max	153.0, 487.0	-442.5, 208.5	183.0, 322.0	-189.0, 16.0		
Week 15						
n	19	18	9	9		
Mean (SD)	252.53 (75.712)	-27.58 (127.045)	215.33 (23.611)	-58.44 (67.259)		
Median	250.00	1.75	219.00	-26.00		
Min, Max	134.0, 444.0	-486.5, 165.5	166.0, 247.0	-168.0, 11.5		

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	20	19	11	11		
Mean (SD)	266.95 (101.946)	-7.41 (93.793)	234.00 (41.788)	-48.14 (71.508)		
Median	230.00	-6.50	239.00	-13.00		
Min, Max	144.0, 522.0	-258.5, 243.5	152.0, 310.0	-182.0, 26.0		
Week 18						
n	19	18	11	11		
Mean (SD)	259.74 (78.101)	-10.27 (88.064)	273.82 (82.084)	-8.32 (62.034)		
Median	260.00	0.25	236.00	-18.00		
Min, Max	163.0, 437.0	-297.5, 113.5	200.0, 461.0	-111.0, 93.5		
Week 20						
n	19	18	11	11		
Mean (SD)	259.11 (88.090)	-17.24 (71.779)	274.45 (94.428)	-7.68 (90.274)		
Median	255.00	-16.00	247.00	1.00		
Min, Max	157.0, 506.0	-228.5, 89.5	185.0, 535.0	-109.0, 229.0		

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	19	18	11	11		
Mean (SD)	277.84 (167.104)	-4.99 (61.104)	270.00 (56.251)	-12.14 (81.069)		
Median	253.00	-12.75	258.00	0.50		
Min, Max	139.0, 906.0	-93.5, 171.5	199.0, 416.0	-145.0, 110.0		
Week 24						
n	18	17	11	11		
Mean (SD)	251.17 (89.281)	-33.02 (132.509)	265.64 (70.129)	-16.50 (89.214)		
Median	214.50	-22.50	247.00	-20.50		
Min, Max	130.0, 468.0	-491.5, 130.5	188.0, 442.0	-125.0, 136.0		
Week 28						
n	18	18	11	11	29	29
Mean (SD)	236.44 (50.462)	-31.94 (118.870)	260.45 (74.381)	-21.68 (90.069)	245.55 (60.518)	-28.05 (107.247)
Median	240.00	-10.50	236.00	-13.00	238.00	-13.00
Min, Max	134.0, 322.0	-467.5, 85.0	173.0, 433.0	-161.0, 127.0	134.0, 433.0	-467.5, 127.0

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	18	17	11	11	29	28
Mean (SD)	269.50 (87.895)	-20.71 (91.187)	254.64 (66.845)	-27.50 (95.936)	263.86 (79.625)	-23.38 (91.366)
Median	247.00	-9.00	241.00	-7.00	241.00	-8.00
Min, Max	150.0, 484.0	-331.5, 101.0	191.0, 421.0	-167.0, 153.5	150.0, 484.0	-331.5, 153.5
Week 36						
n	18	17	9	9	27	26
Mean (SD)	238.78 (57.702)	-12.31 (50.948)	273.89 (86.867)	7.00 (66.997)	250.48 (69.161)	-5.63 (56.439)
Median	235.00	-16.50	233.00	-2.00	233.00	-13.50
Min, Max	131.0, 359.0	-86.0, 111.7	206.0, 478.0	-61.0, 172.0	131.0, 478.0	-86.0, 172.0
Week 40						
n	17	16	11	11	28	27
Mean (SD)	254.76 (55.459)	-24.24 (104.999)	276.09 (66.532)	-6.05 (85.011)	263.14 (59.788)	-16.83 (96.036)
Median	251.00	0.08	265.00	-18.00	254.50	-6.00
Min, Max	170.0, 339.0	-395.5, 54.5	177.0, 394.0	-120.0, 120.0	170.0, 394.0	-395.5, 120.0

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	16	15	10	10	26	25
Mean (SD)	240.44 (56.068)	-39.09 (120.224)	255.10 (50.825)	-18.05 (74.105)	246.08 (53.563)	-30.67 (102.963)
Median	231.50	-25.00	249.50	0.25	244.00	-20.50
Min, Max	149.0, 318.0	-443.5, 93.0	176.0, 356.0	-118.0, 68.0	149.0, 356.0	-443.5, 93.0
Week 48						
n	12	12	11	11	23	23
Mean (SD)	262.33 (78.336)	15.17 (58.094)	252.91 (57.185)	-29.23 (65.579)	257.83 (67.660)	-6.07 (64.470)
Median	244.50	-6.25	243.00	-16.50	243.00	-8.50
Min, Max	157.0, 405.0	-67.5, 126.5	170.0, 351.0	-164.0, 36.0	157.0, 405.0	-164.0, 126.5
Week 52						
n	13	13	11	11	24	24
Mean (SD)	246.00 (68.223)	-38.65 (126.003)	266.91 (69.703)	-15.23 (92.051)	255.58 (68.220)	-27.92 (110.045)
Median	227.00	-24.00	247.00	-4.00	241.50	-17.25
Min, Max	155.0, 387.0	-436.5, 82.5	183.0, 455.0	-189.0, 149.0	155.0, 455.0	-436.5, 149.0

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	13	13	11	11	24	24
Mean (SD)	264.54 (77.671)	-20.12 (99.597)	319.00 (131.416)	36.86 (124.386)	289.50 (106.886)	6.00 (112.886)
Median	251.00	-6.50	288.00	24.00	252.00	-2.75
Min, Max	153.0, 405.0	-329.5, 85.5	204.0, 673.0	-125.0, 339.0	153.0, 673.0	-329.5, 339.0
Week 60						
n	11	11	9	9	20	20
Mean (SD)	247.09 (67.747)	7.00 (41.914)	284.78 (81.092)	7.94 (71.535)	264.05 (74.528)	7.43 (55.493)
Median	237.00	1.50	243.00	6.50	242.50	4.00
Min, Max	161.0, 391.0	-72.5, 71.0	175.0, 444.0	-119.0, 110.0	161.0, 444.0	-119.0, 110.0
Week 64						
n	9	9	8	8	17	17
Mean (SD)	245.33 (62.791)	4.78 (33.468)	249.75 (72.221)	-23.44 (70.760)	247.41 (65.257)	-8.50 (54.419)
Median	230.00	-6.50	230.00	-16.75	230.00	-14.00
Min, Max	151.0, 334.0	-29.5, 61.5	193.0, 421.0	-131.5, 87.0	151.0, 421.0	-131.5, 87.0

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Post-hoc Analysen Studie ALPHA

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Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	8	8	7	7	15	15
Mean (SD)	255.50 (55.201)	19.69 (30.075)	228.29 (33.245)	-36.21 (71.478)	242.80 (46.848)	-6.40 (58.951)
Median	276.50	22.00	233.00	-12.00	258.00	14.00
Min, Max	143.0, 306.0	-22.5, 64.0	172.0, 267.0	-131.5, 44.0	143.0, 306.0	-131.5, 64.0
Week 72						
n	6	6	7	7	13	13
Mean (SD)	220.33 (60.513)	2.17 (37.336)	235.00 (34.545)	-29.50 (64.795)	228.23 (46.694)	-14.88 (54.314)
Median	213.50	-11.00	245.00	-16.00	236.00	-16.00
Min, Max	142.0, 312.0	-30.0, 69.5	162.0, 264.0	-132.0, 35.0	142.0, 312.0	-132.0, 69.5
Week 80						
n	5	5	4	4	9	9
Mean (SD)	232.00 (58.254)	13.00 (39.987)	238.00 (50.957)	-31.25 (42.148)	234.67 (51.774)	-6.67 (44.828)
Median	217.00	11.50	243.00	-38.75	242.00	-12.50
Min, Max	153.0, 304.0	-37.5, 61.5	171.0, 295.0	-72.5, 25.0	153.0, 304.0	-72.5, 61.5

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 Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
 D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
 LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.
 Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	4	4	4	4	8	8
Mean (SD)	247.25 (52.201)	16.50 (26.546)	256.75 (25.591)	-15.63 (57.695)	252.00 (38.396)	0.44 (44.983)
Median	259.50	22.50	267.00	-6.50	267.00	7.00
Min, Max	176.0, 294.0	-18.5, 39.5	219.0, 274.0	-93.5, 44.0	176.0, 294.0	-93.5, 44.0
Week 96						
n	2	2	4	4	6	6
Mean (SD)	207.50 (60.104)	-5.50 (7.071)	262.00 (70.763)	-10.38 (59.288)	243.83 (67.223)	-8.75 (46.102)
Median	207.50	-5.50	261.50	-33.00	238.50	-18.00
Min, Max	165.0, 250.0	-10.5, -0.5	183.0, 342.0	-52.5, 77.0	165.0, 342.0	-52.5, 77.0
Week 104						
n	0	0	1	1	1	1
Mean (SD)			291.00 (NA)	-76.50 (NA)	291.00 (NA)	-76.50 (NA)
Median			291.00	-76.50	291.00	-76.50
Min, Max			291.0, 291.0	-76.5, -76.5	291.0, 291.0	-76.5, -76.5

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		13		49	
Mean (SD)	318.54 (124.525)		259.96 (83.620)		303.00 (117.208)	
Median	295.00		247.00		275.00	
Min, Max	140.0, 809.0		139.0, 404.0		139.0, 809.0	
Week 1						
n	33	33	14	13		
Mean (SD)	251.55 (77.863)	-73.92 (73.761)	317.57 (277.759)	-14.88 (32.623)		
Median	241.00	-60.00	242.00	-13.00		
Min, Max	147.0, 532.0	-277.0, 87.0	128.0, 1260.0	-72.5, 56.0		
Week 2						
n	36	36	14	13		
Mean (SD)	241.58 (73.778)	-76.96 (83.512)	323.36 (277.127)	-8.65 (38.440)		
Median	228.00	-63.50	247.00	0.50		
Min, Max	117.0, 515.0	-307.5, 66.5	144.0, 1260.0	-73.0, 76.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	34	34	14	13		
Mean (SD)	236.18 (79.990)	-83.60 (80.120)	309.00 (280.436)	-24.12 (30.833)		
Median	222.00	-64.50	235.00	-10.50		
Min, Max	122.0, 540.0	-301.5, 9.0	123.0, 1260.0	-83.0, 6.0		
Week 4						
n	35	35	14	13		
Mean (SD)	264.00 (82.670)	-54.06 (81.733)	321.57 (278.467)	-10.58 (28.074)		
Median	261.00	-40.00	261.00	-15.00		
Min, Max	138.0, 551.0	-258.0, 114.0	123.0, 1260.0	-82.5, 17.5		
Week 6						
n	34	34	14	13		
Mean (SD)	269.03 (95.782)	-50.87 (80.006)	339.43 (281.598)	8.65 (61.208)		
Median	254.00	-47.50	257.50	-4.00		
Min, Max	146.0, 698.0	-238.5, 142.0	130.0, 1260.0	-98.5, 125.0		

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	34	34	14	13		
Mean (SD)	284.50 (94.015)	-34.63 (68.858)	336.79 (280.608)	5.81 (51.852)		
Median	259.00	-36.00	255.00	10.00		
Min, Max	146.0, 543.0	-266.0, 88.5	122.0, 1260.0	-113.5, 92.5		
Week 10						
n	32	32	14	13		
Mean (SD)	267.59 (80.993)	-50.55 (77.793)	318.79 (280.087)	-13.58 (38.645)		
Median	259.00	-27.25	233.50	-4.00		
Min, Max	158.0, 589.0	-220.0, 44.5	155.0, 1260.0	-98.5, 44.0		
Week 12						
n	34	34	14	13		
Mean (SD)	275.18 (72.489)	-40.01 (89.962)	317.79 (280.841)	-14.65 (30.204)		
Median	271.50	-15.50	241.50	-7.50		
Min, Max	142.0, 519.0	-290.0, 70.5	128.0, 1260.0	-84.5, 25.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	35	35	12	11		
Mean (SD)	268.69 (88.874)	-47.71 (85.081)	261.00 (195.238)	-34.36 (46.418)		
Median	253.00	-12.50	212.50	-24.50		
Min, Max	146.0, 639.0	-259.5, 133.5	122.0, 854.0	-128.0, 27.5		
Week 14						
n	35	35	13	12		
Mean (SD)	277.51 (97.264)	-38.89 (98.173)	241.46 (96.056)	-31.21 (47.720)		
Median	260.00	-25.00	224.00	-10.75		
Min, Max	135.0, 587.0	-243.5, 214.5	118.0, 501.0	-135.5, 22.5		
Week 15						
n	32	32	13	12		
Mean (SD)	278.16 (102.431)	-40.45 (104.031)	222.54 (66.654)	-40.04 (47.811)		
Median	254.00	-29.00	234.00	-27.25		
Min, Max	142.0, 627.0	-243.5, 283.5	123.0, 361.0	-127.0, 29.0		

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Post-hoc Analysen Studie ALPHA

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Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	34	34	13	12		
Mean (SD)	295.71 (106.783)	-20.53 (113.334)	256.46 (89.093)	0.29 (90.779)		
Median	277.00	-19.00	254.00	-16.00		
Min, Max	159.0, 639.0	-239.5, 332.0	121.0, 439.0	-97.5, 252.0		
Week 18						
n	35	35	12	11		
Mean (SD)	270.83 (81.729)	-45.57 (86.209)	247.83 (60.823)	-5.45 (54.145)		
Median	262.00	-23.00	282.50	0.00		
Min, Max	159.0, 568.0	-241.0, 88.0	141.0, 309.0	-105.0, 67.5		
Week 20						
n	30	30	13	12		
Mean (SD)	296.50 (119.875)	-34.67 (89.643)	258.92 (67.927)	0.46 (57.833)		
Median	264.00	-27.50	277.00	-1.50		
Min, Max	171.0, 725.0	-243.5, 235.5	137.0, 348.0	-127.0, 84.5		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

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Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	35	35	13	12		
Mean (SD)	262.34 (74.004)	-54.06 (85.755)	261.85 (63.193)	4.96 (71.618)		
Median	261.00	-32.00	286.00	16.00		
Min, Max	108.0, 502.0	-307.0, 74.5	169.0, 349.0	-180.0, 83.5		
Week 24						
n	34	34	12	11		
Mean (SD)	289.74 (92.021)	-29.35 (92.888)	294.42 (67.770)	28.23 (68.303)		
Median	275.50	-9.50	297.50	51.00		
Min, Max	139.0, 573.0	-303.0, 142.0	183.0, 394.0	-116.0, 135.5		
Week 28						
n	33	33	12	11	45	44
Mean (SD)	291.39 (106.650)	-29.11 (108.988)	296.00 (58.737)	31.23 (71.609)	292.62 (95.598)	-14.02 (103.589)
Median	275.00	-28.00	302.00	27.50	282.00	-6.50
Min, Max	156.0, 632.0	-218.0, 370.5	195.0, 405.0	-104.0, 143.5	156.0, 632.0	-218.0, 370.5

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Post-hoc Analysen Studie ALPHA

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Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	30	30	11	10	41	40
Mean (SD)	283.37 (88.335)	-39.47 (92.869)	292.27 (88.357)	21.40 (90.021)	285.76 (87.321)	-24.25 (94.846)
Median	259.50	-6.50	267.00	28.25	267.00	-2.00
Min, Max	165.0, 512.0	-297.0, 79.5	154.0, 421.0	-137.0, 141.0	154.0, 512.0	-297.0, 141.0
Week 36						
n	29	29	11	10	40	39
Mean (SD)	289.03 (116.597)	-21.93 (126.082)	288.55 (79.075)	20.30 (61.381)	288.90 (106.601)	-11.10 (113.819)
Median	257.00	-26.00	294.00	25.75	272.50	-14.50
Min, Max	168.0, 804.0	-229.5, 470.0	166.0, 426.0	-62.5, 107.0	166.0, 804.0	-229.5, 470.0
Week 40						
n	29	29	10	9	39	38
Mean (SD)	300.03 (93.149)	-14.10 (105.089)	266.60 (73.733)	-9.67 (66.033)	291.46 (88.880)	-13.05 (96.456)
Median	278.00	-9.00	253.50	-7.50	277.00	-8.50
Min, Max	203.0, 623.0	-230.5, 209.0	170.0, 376.0	-164.0, 53.0	170.0, 623.0	-230.5, 209.0

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	27	27	10	9	37	36
Mean (SD)	274.96 (54.592)	-38.54 (65.378)	273.50 (66.074)	-5.50 (79.711)	274.57 (56.959)	-30.28 (69.556)
Median	278.00	-25.00	288.00	17.00	282.00	-19.00
Min, Max	182.0, 394.0	-210.5, 78.0	164.0, 373.0	-180.0, 81.0	164.0, 394.0	-210.5, 81.0
Week 48						
n	24	24	9	8	33	32
Mean (SD)	293.83 (68.762)	-18.63 (87.206)	298.56 (76.043)	26.19 (66.845)	295.12 (69.632)	-7.42 (83.905)
Median	280.00	-14.25	316.00	36.00	288.00	-9.00
Min, Max	173.0, 455.0	-198.5, 230.0	173.0, 413.0	-88.0, 125.5	173.0, 455.0	-198.5, 230.0
Week 52						
n	22	22	8	7	30	29
Mean (SD)	276.64 (55.417)	-34.23 (64.338)	271.88 (69.934)	-8.14 (83.287)	275.37 (58.386)	-27.93 (68.702)
Median	280.50	-27.00	265.00	18.00	272.00	-23.00
Min, Max	184.0, 360.0	-170.5, 48.5	175.0, 368.0	-138.0, 100.0	175.0, 368.0	-170.5, 100.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	23	23	7	6	30	29
Mean (SD)	314.57 (100.588)	2.52 (123.912)	269.14 (63.654)	-19.00 (83.906)	303.97 (94.318)	-1.93 (115.758)
Median	287.00	-3.50	256.00	6.25	282.50	-3.50
Min, Max	161.0, 635.0	-233.5, 339.0	186.0, 381.0	-149.0, 69.0	161.0, 635.0	-233.5, 339.0
Week 60						
n	20	20	7	6	27	26
Mean (SD)	273.90 (63.645)	-42.60 (91.593)	265.43 (70.282)	-22.50 (78.187)	271.70 (64.143)	-37.96 (87.596)
Median	280.00	-45.25	253.00	-1.50	280.00	-39.00
Min, Max	181.0, 444.0	-199.5, 204.0	166.0, 376.0	-151.0, 51.0	166.0, 444.0	-199.5, 204.0
Week 64						
n	19	19	6	5	25	24
Mean (SD)	292.16 (49.391)	-27.18 (85.826)	281.83 (87.413)	8.10 (55.788)	289.68 (58.666)	-19.83 (80.748)
Median	299.00	-25.50	284.00	34.00	299.00	-20.25
Min, Max	186.0, 366.0	-227.5, 84.0	167.0, 390.0	-67.0, 57.5	167.0, 390.0	-227.5, 84.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	15	15	5	5	20	20
Mean (SD)	278.27 (62.935)	-23.77 (85.782)	252.80 (71.356)	0.70 (81.467)	271.90 (64.175)	-17.65 (83.292)
Median	284.00	-41.00	272.00	22.00	280.50	-18.75
Min, Max	137.0, 412.0	-192.0, 99.5	162.0, 343.0	-119.0, 85.0	137.0, 412.0	-192.0, 99.5
Week 72						
n	13	13	4	4	17	17
Mean (SD)	273.62 (56.538)	-27.42 (96.464)	237.75 (59.005)	-5.50 (106.425)	265.18 (57.412)	-22.26 (95.888)
Median	269.00	16.00	231.00	-2.00	268.00	16.00
Min, Max	192.0, 403.0	-206.5, 78.0	182.0, 307.0	-138.0, 120.0	182.0, 403.0	-206.5, 120.0
Week 80						
n	12	12	4	4	16	16
Mean (SD)	295.83 (68.756)	-9.54 (107.647)	248.25 (97.609)	5.00 (31.927)	283.94 (76.323)	-5.91 (93.509)
Median	295.50	-14.75	213.00	-8.50	287.00	-10.50
Min, Max	188.0, 449.0	-162.5, 215.5	178.0, 389.0	-15.0, 52.0	178.0, 449.0	-162.5, 215.5

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2x$ ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.7

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	7	3	3	10	10
Mean (SD)	338.86 (97.469)	63.36 (132.725)	223.00 (19.925)	33.33 (25.027)	304.10 (97.743)	54.35 (109.970)
Median	350.00	21.50	212.00	32.00	271.00	26.75
Min, Max	239.0, 529.0	-91.0, 329.0	211.0, 246.0	9.0, 59.0	211.0, 529.0	-91.0, 329.0
Week 96						
n	5	5	2	2	7	7
Mean (SD)	267.40 (53.654)	-24.20 (64.376)	204.00 (36.770)	9.50 (47.376)	249.29 (55.692)	-14.57 (58.373)
Median	246.00	-17.50	204.00	9.50	230.00	-17.50
Min, Max	220.0, 348.0	-105.0, 61.5	178.0, 230.0	-24.0, 43.0	178.0, 348.0	-105.0, 61.5
Week 104						
n	1	1	1	1	2	2
Mean (SD)	193.00 (NA)	-44.50 (NA)	221.00 (NA)	34.00 (NA)	207.00 (19.799)	-5.25 (55.508)
Median	193.00	-44.50	221.00	34.00	207.00	-5.25
Min, Max	193.0, 193.0	-44.5, -44.5	221.0, 221.0	34.0, 34.0	193.0, 221.0	-44.5, 34.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.8

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		3		11	
Mean (SD)	324.06 (90.909)		225.17 (36.295)		297.09 (90.457)	
Median	313.50		230.50		258.50	
Min, Max	204.0, 473.0		186.5, 258.5		186.5, 473.0	
Week 1						
n	7	7	4	3		
Mean (SD)	292.00 (73.709)	-42.36 (27.369)	481.00 (519.636)	-3.83 (14.572)		
Median	287.00	-42.00	233.00	-5.50		
Min, Max	206.0, 396.0	-77.0, 2.0	198.0, 1260.0	-17.5, 11.5		
Week 2						
n	8	8	4	3		
Mean (SD)	284.38 (58.751)	-39.69 (62.281)	485.75 (516.900)	2.50 (2.646)		
Median	298.00	-41.00	245.50	1.50		
Min, Max	193.0, 354.0	-119.0, 53.0	192.0, 1260.0	0.5, 5.5		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2x$ ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.8.8

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	8	8	4	3		
Mean (SD)	277.88 (64.625)	-46.19 (50.909)	482.00 (519.549)	-2.50 (7.000)		
Median	268.50	-54.50	240.50	0.50		
Min, Max	189.0, 370.0	-103.5, 24.0	187.0, 1260.0	-10.5, 2.5		
Week 4						
n	8	8	4	3		
Mean (SD)	266.38 (50.034)	-57.69 (63.863)	495.25 (510.787)	15.17 (2.082)		
Median	268.50	-34.50	260.50	14.50		
Min, Max	209.0, 336.0	-147.0, 16.0	200.0, 1260.0	13.5, 17.5		
Week 6						
n	8	8	4	3		
Mean (SD)	280.38 (50.466)	-43.69 (73.009)	485.00 (517.792)	1.50 (11.000)		
Median	266.50	-3.00	246.00	1.50		
Min, Max	205.0, 338.0	-148.0, 28.0	188.0, 1260.0	-9.5, 12.5		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.8.8

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	7	7	4	3		
Mean (SD)	291.43 (74.895)	-24.71 (55.916)	484.75 (517.372)	1.17 (16.010)		
Median	298.00	-3.00	237.50	0.50		
Min, Max	198.0, 394.0	-110.0, 32.0	204.0, 1260.0	-14.5, 17.5		
Week 10						
n	8	8	4	3		
Mean (SD)	290.88 (67.433)	-33.19 (78.019)	477.25 (522.046)	-8.83 (19.502)		
Median	301.00	-7.25	224.50	-17.50		
Min, Max	209.0, 365.0	-177.0, 45.0	200.0, 1260.0	-22.5, 13.5		
Week 12						
n	8	8	4	3		
Mean (SD)	295.13 (76.219)	-28.94 (65.368)	480.50 (520.305)	-4.50 (5.196)		
Median	276.50	-5.50	237.00	-7.50		
Min, Max	222.0, 453.0	-157.0, 22.0	188.0, 1260.0	-7.5, 1.5		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.8.8

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	8	8	4	3		
Mean (SD)	263.38 (53.772)	-60.69 (75.795)	360.25 (330.118)	-29.50 (23.896)		
Median	243.50	-32.00	212.50	-24.50		
Min, Max	202.0, 362.0	-186.0, 27.0	162.0, 854.0	-55.5, -8.5		
Week 14						
n	8	8	4	3		
Mean (SD)	273.50 (61.762)	-50.56 (68.400)	285.00 (149.766)	-12.17 (14.364)		
Median	262.50	-21.50	240.50	-6.50		
Min, Max	210.0, 379.0	-187.0, 18.0	158.0, 501.0	-28.5, -1.5		
Week 15						
n	8	8	4	3		
Mean (SD)	263.38 (71.702)	-60.69 (72.548)	251.75 (80.479)	-9.83 (18.502)		
Median	233.00	-24.50	239.50	-19.50		
Min, Max	201.0, 418.0	-198.0, -3.0	167.0, 361.0	-21.5, 11.5		

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.8.8

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	8	8	4	3		
Mean (SD)	324.63 (139.527)	0.56 (148.791)	256.25 (49.426)	10.50 (15.000)		
Median	292.50	-13.50	255.00	10.50		
Min, Max	195.0, 639.0	-142.5, 332.0	197.0, 318.0	-4.5, 25.5		
Week 18						
n	8	8	4	3		
Mean (SD)	266.38 (61.822)	-57.69 (77.724)	282.25 (22.485)	48.17 (21.197)		
Median	248.00	-25.50	283.00	51.50		
Min, Max	200.0, 374.0	-188.0, 10.0	254.0, 309.0	25.5, 67.5		
Week 20						
n	8	8	4	3		
Mean (SD)	277.00 (69.453)	-47.06 (56.269)	292.00 (67.404)	48.17 (38.682)		
Median	265.50	-38.50	313.00	52.50		
Min, Max	201.0, 434.0	-142.0, 23.0	194.0, 348.0	7.5, 84.5		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.8

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	8	8	4	3		
Mean (SD)	267.63 (60.891)	-56.44 (62.720)	301.25 (36.124)	65.83 (16.258)		
Median	258.00	-47.25	312.00	62.50		
Min, Max	175.0, 359.0	-172.0, 15.0	249.0, 332.0	51.5, 83.5		
Week 24						
n	8	8	4	3		
Mean (SD)	263.38 (57.495)	-60.69 (60.740)	306.25 (83.703)	66.83 (69.515)		
Median	262.00	-50.50	338.00	68.50		
Min, Max	187.0, 361.0	-161.0, 19.0	183.0, 366.0	-3.5, 135.5		
Week 28						
n	8	8	4	3	12	11
Mean (SD)	279.00 (91.935)	-45.06 (68.807)	323.25 (25.316)	94.17 (59.911)	293.75 (77.640)	-7.09 (90.892)
Median	244.00	-14.50	332.50	111.50	291.50	-8.00
Min, Max	203.0, 486.0	-176.0, 13.0	286.0, 342.0	27.5, 143.5	203.0, 486.0	-176.0, 143.5

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2x$ ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.8

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	8	8	3	2	11	10
Mean (SD)	298.00 (99.529)	-26.06 (70.069)	263.67 (102.861)	-6.00 (37.477)	288.64 (96.475)	-22.05 (63.610)
Median	277.00	-6.50	279.00	-6.00	279.00	-6.50
Min, Max	189.0, 467.0	-185.0, 38.5	154.0, 358.0	-32.5, 20.5	154.0, 467.0	-185.0, 38.5
Week 36						
n	7	7	3	2	10	9
Mean (SD)	290.43 (78.066)	-43.93 (66.358)	270.00 (90.266)	18.50 (55.154)	284.30 (77.272)	-30.06 (66.638)
Median	302.00	-56.00	316.00	18.50	309.00	-20.50
Min, Max	193.0, 404.0	-164.0, 39.0	166.0, 328.0	-20.5, 57.5	166.0, 404.0	-164.0, 57.5
Week 40						
n	6	6	3	2	9	8
Mean (SD)	309.17 (110.688)	-46.92 (151.132)	279.33 (91.139)	18.00 (36.062)	299.22 (99.782)	-30.69 (131.923)
Median	280.00	-65.75	302.00	18.00	289.00	-15.25
Min, Max	215.0, 516.0	-193.0, 209.0	179.0, 357.0	-7.5, 43.5	179.0, 516.0	-193.0, 209.0

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	5	5	2	1	7	6
Mean (SD)	301.20 (26.329)	-44.50 (72.498)	318.50 (13.435)	50.50 (NA)	306.14 (23.738)	-28.67 (75.558)
Median	295.00	-25.00	318.50	50.50	309.00	-4.50
Min, Max	265.0, 329.0	-148.0, 22.0	309.0, 328.0	50.5, 50.5	265.0, 329.0	-148.0, 50.5
Week 48						
n	4	4	2	1	6	5
Mean (SD)	291.50 (85.098)	-63.88 (87.818)	345.00 (14.142)	76.50 (NA)	309.33 (71.751)	-35.80 (98.616)
Median	309.00	-76.75	345.00	76.50	325.50	-76.00
Min, Max	173.0, 375.0	-157.0, 55.0	335.0, 355.0	76.5, 76.5	173.0, 375.0	-157.0, 76.5
Week 52						
n	4	4	1	0	5	4
Mean (SD)	317.25 (74.652)	-38.13 (77.686)	368.00 (NA)		327.40 (68.519)	-38.13 (77.686)
Median	332.00	-53.25	368.00		360.00	-53.25
Min, Max	218.0, 387.0	-113.0, 67.0	368.0, 368.0		218.0, 387.0	-113.0, 67.0

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.8

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	4	4	1	0	5	4
Mean (SD)	300.50 (75.492)	-54.88 (91.099)	381.00 (NA)		316.60 (74.634)	-54.88 (91.099)
Median	304.50	-69.75	381.00		327.00	-69.75
Min, Max	207.0, 386.0	-146.0, 66.0	381.0, 381.0		207.0, 386.0	-146.0, 66.0
Week 60						
n	4	4	1	0	5	4
Mean (SD)	286.25 (88.225)	-69.13 (100.855)	376.00 (NA)		304.20 (86.306)	-69.13 (100.855)
Median	286.50	-93.25	376.00		312.00	-93.25
Min, Max	181.0, 391.0	-161.0, 71.0	376.0, 376.0		181.0, 391.0	-161.0, 71.0
Week 64						
n	4	4	1	0	5	4
Mean (SD)	291.50 (73.105)	-63.88 (72.701)	390.00 (NA)		311.20 (77.128)	-63.88 (72.701)
Median	313.00	-44.25	390.00		319.00	-44.25
Min, Max	186.0, 354.0	-166.0, -1.0	390.0, 390.0		186.0, 390.0	-166.0, -1.0

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.8

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Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	3	3	0	0	3	3
Mean (SD)	266.67 (78.749)	-49.50 (31.277)			266.67 (78.749)	-49.50 (31.277)
Median	306.00	-61.50			306.00	-61.50
Min, Max	176.0, 318.0	-73.0, -14.0			176.0, 318.0	-73.0, -14.0
Week 72						
n	1	1	0	0	1	1
Mean (SD)	192.00 (NA)	-57.00 (NA)			192.00 (NA)	-57.00 (NA)
Median	192.00	-57.00			192.00	-57.00
Min, Max	192.0, 192.0	-57.0, -57.0			192.0, 192.0	-57.0, -57.0

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	48		22		70	
Mean (SD)	300.65 (128.710)		286.98 (87.101)		296.35 (116.765)	
Median	259.00		277.50		261.00	
Min, Max	140.0, 809.0		139.0, 506.0		139.0, 809.0	
Week 1						
n	44	43	21	21		
Mean (SD)	239.77 (80.698)	-69.53 (77.215)	266.95 (75.318)	-22.48 (89.079)		
Median	222.50	-49.50	250.00	-13.00		
Min, Max	137.0, 532.0	-295.5, 87.0	128.0, 404.0	-332.0, 155.0		
Week 2						
n	49	48	22	22		
Mean (SD)	242.18 (128.018)	-73.28 (81.072)	300.36 (211.162)	13.39 (226.214)		
Median	222.00	-50.00	261.50	-11.50		
Min, Max	117.0, 953.0	-307.5, 66.5	144.0, 1195.0	-345.0, 955.0		

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Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	46	45	20	20		
Mean (SD)	223.48 (75.172)	-82.04 (87.871)	267.25 (85.971)	-11.35 (84.170)		
Median	216.50	-54.00	262.50	-11.50		
Min, Max	121.0, 540.0	-398.5, 9.5	123.0, 433.0	-185.0, 193.0		
Week 4						
n	48	47	18	18		
Mean (SD)	264.21 (124.689)	-48.10 (81.619)	270.61 (71.469)	0.28 (37.289)		
Median	256.50	-36.00	270.00	1.00		
Min, Max	108.0, 847.0	-259.5, 114.0	123.0, 389.0	-82.5, 76.5		
Week 6						
n	46	45	21	21		
Mean (SD)	271.89 (125.486)	-36.63 (73.625)	302.38 (151.243)	25.83 (150.250)		
Median	250.00	-27.33	258.00	1.00		
Min, Max	124.0, 794.0	-238.5, 142.0	130.0, 843.0	-167.0, 603.0		

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Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	47	46	20	20		
Mean (SD)	282.64 (108.452)	-23.12 (90.538)	304.35 (118.051)	32.58 (106.462)		
Median	257.00	-26.75	276.50	15.50		
Min, Max	145.0, 629.0	-337.5, 255.5	122.0, 672.0	-113.5, 432.0		
Week 10						
n	43	42	21	21		
Mean (SD)	257.88 (74.664)	-29.51 (71.176)	276.19 (85.120)	-0.36 (79.149)		
Median	248.00	-17.75	258.00	8.50		
Min, Max	148.0, 589.0	-220.0, 82.5	155.0, 459.0	-137.0, 191.5		
Week 12						
n	44	43	21	21		
Mean (SD)	260.00 (69.518)	-36.32 (98.050)	277.62 (75.782)	1.07 (41.725)		
Median	262.00	-13.50	255.00	6.00		
Min, Max	135.0, 519.0	-379.5, 99.5	128.0, 410.0	-84.5, 80.0		

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Post-hoc Analysen Studie ALPHA

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Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	45	44	19	19		
Mean (SD)	273.56 (127.751)	-25.02 (144.739)	213.21 (48.752)	-54.37 (65.063)		
Median	253.00	-8.25	219.00	-31.00		
Min, Max	129.0, 882.0	-496.5, 627.5	122.0, 315.0	-196.0, 27.5		
Week 14						
n	45	44	19	19		
Mean (SD)	273.09 (100.095)	-24.46 (118.701)	223.42 (50.672)	-45.34 (63.777)		
Median	260.00	-14.00	220.00	-15.00		
Min, Max	135.0, 587.0	-442.5, 214.5	118.0, 322.0	-189.0, 22.5		
Week 15						
n	43	42	18	18		
Mean (SD)	269.58 (97.593)	-31.08 (117.811)	212.44 (44.433)	-54.28 (58.064)		
Median	255.00	-16.75	216.00	-34.00		
Min, Max	134.0, 627.0	-486.5, 283.5	123.0, 279.0	-168.0, 29.0		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.8

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	46	45	20	20		
Mean (SD)	278.17 (98.114)	-18.74 (98.491)	244.15 (75.360)	-27.88 (89.134)		
Median	260.00	-11.50	240.00	-18.00		
Min, Max	144.0, 582.0	-258.5, 243.5	121.0, 439.0	-182.0, 252.0		
Week 18						
n	46	45	19	19		
Mean (SD)	267.02 (83.221)	-29.30 (89.389)	255.63 (77.491)	-15.58 (55.985)		
Median	263.00	-8.00	236.00	-18.00		
Min, Max	159.0, 568.0	-297.5, 113.5	141.0, 461.0	-111.0, 93.5		
Week 20						
n	41	40	20	20		
Mean (SD)	282.98 (116.046)	-24.35 (87.478)	260.85 (82.433)	-11.18 (74.863)		
Median	257.00	-12.25	248.50	-5.50		
Min, Max	157.0, 725.0	-243.5, 235.5	137.0, 535.0	-127.0, 229.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:34

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.8.8

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	46	45	20	20		
Mean (SD)	267.83 (121.640)	-34.01 (84.095)	258.45 (60.670)	-13.58 (74.989)		
Median	255.00	-18.50	257.00	0.25		
Min, Max	108.0, 906.0	-307.0, 171.5	169.0, 416.0	-180.0, 110.0		
Week 24						
n	44	43	19	19		
Mean (SD)	278.75 (97.374)	-24.97 (112.482)	275.26 (66.776)	-3.76 (79.815)		
Median	268.50	-5.00	257.00	11.00		
Min, Max	130.0, 573.0	-491.5, 142.0	188.0, 442.0	-125.0, 136.0		
Week 28						
n	43	43	19	19	62	62
Mean (SD)	270.70 (95.469)	-27.32 (118.034)	269.68 (70.365)	-9.34 (79.184)	270.39 (87.959)	-21.81 (107.297)
Median	267.00	-24.50	254.00	1.00	262.00	-13.00
Min, Max	134.0, 632.0	-467.5, 370.5	173.0, 433.0	-161.0, 127.0	134.0, 632.0	-467.5, 370.5

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.8

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	40	39	19	19	59	58
Mean (SD)	274.20 (85.735)	-34.04 (96.271)	275.00 (77.835)	-4.03 (98.907)	274.46 (82.601)	-24.21 (97.313)
Median	253.00	-11.50	250.00	9.00	250.00	-5.00
Min, Max	150.0, 512.0	-331.5, 101.0	191.0, 421.0	-167.0, 153.5	150.0, 512.0	-331.5, 153.5
Week 36						
n	40	39	17	17	57	56
Mean (SD)	266.18 (104.420)	-13.79 (109.523)	284.06 (81.838)	13.47 (64.984)	271.51 (97.853)	-5.51 (98.367)
Median	255.50	-16.50	256.00	-2.00	256.00	-13.25
Min, Max	131.0, 804.0	-229.5, 470.0	172.0, 478.0	-62.5, 172.0	131.0, 804.0	-229.5, 470.0
Week 40						
n	40	39	18	18	58	57
Mean (SD)	279.43 (79.783)	-13.21 (96.728)	270.28 (67.232)	-10.53 (78.385)	276.59 (75.641)	-12.37 (90.641)
Median	264.00	-0.50	258.50	-19.25	262.00	-8.00
Min, Max	170.0, 623.0	-395.5, 153.0	170.0, 394.0	-164.0, 120.0	170.0, 623.0	-395.5, 153.0

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 Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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 Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.8

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	38	37	18	18	56	55
Mean (SD)	256.97 (58.171)	-37.95 (90.104)	258.28 (58.171)	-15.58 (75.484)	257.39 (57.643)	-30.63 (85.548)
Median	266.50	-25.00	249.50	5.75	259.00	-19.50
Min, Max	149.0, 394.0	-443.5, 93.0	164.0, 373.0	-180.0, 81.0	149.0, 394.0	-443.5, 93.0
Week 48						
n	32	32	18	18	50	50
Mean (SD)	282.31 (72.326)	-0.30 (76.972)	265.50 (67.605)	-10.47 (69.172)	276.26 (70.439)	-3.96 (73.706)
Median	275.00	-8.50	257.00	-1.00	273.00	-8.50
Min, Max	157.0, 455.0	-198.5, 230.0	170.0, 413.0	-164.0, 125.5	157.0, 455.0	-198.5, 230.0
Week 52						
n	31	31	18	18	49	49
Mean (SD)	258.55 (57.470)	-35.58 (93.002)	263.50 (65.345)	-12.47 (86.286)	260.37 (59.853)	-27.09 (90.385)
Median	256.00	-23.00	251.50	7.00	256.00	-16.00
Min, Max	155.0, 360.0	-436.5, 82.5	175.0, 455.0	-189.0, 149.0	155.0, 455.0	-436.5, 149.0

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.8

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	32	32	17	17	49	49
Mean (SD)	296.00 (98.145)	0.50 (117.128)	294.82 (111.980)	17.15 (112.371)	295.59 (101.986)	6.28 (114.600)
Median	281.50	-3.75	256.00	24.00	272.00	-1.50
Min, Max	153.0, 635.0	-329.5, 339.0	186.0, 673.0	-149.0, 339.0	153.0, 673.0	-329.5, 339.0
Week 60						
n	27	27	15	15	42	42
Mean (SD)	261.15 (62.762)	-18.46 (77.203)	269.67 (72.269)	-4.23 (73.115)	264.19 (65.562)	-13.38 (75.184)
Median	259.00	-10.50	243.00	6.50	252.50	-7.50
Min, Max	161.0, 444.0	-199.5, 204.0	166.0, 444.0	-151.0, 110.0	161.0, 444.0	-199.5, 204.0
Week 64						
n	24	24	13	13	37	37
Mean (SD)	274.71 (55.889)	-9.08 (72.724)	253.77 (71.305)	-11.31 (64.909)	267.35 (61.588)	-9.86 (69.170)
Median	276.50	-10.50	231.00	-14.00	261.00	-14.00
Min, Max	151.0, 366.0	-227.5, 84.0	167.0, 421.0	-131.5, 87.0	151.0, 421.0	-227.5, 87.0

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Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.8

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	20	20	12	12	32	32
Mean (SD)	270.90 (59.399)	-2.53 (76.602)	238.50 (51.125)	-20.83 (74.576)	258.75 (57.826)	-9.39 (75.173)
Median	277.50	19.50	234.50	1.00	272.50	15.75
Min, Max	137.0, 412.0	-192.0, 99.5	162.0, 343.0	-131.5, 85.0	137.0, 412.0	-192.0, 99.5
Week 72						
n	18	18	11	11	29	29
Mean (SD)	260.39 (61.306)	-15.92 (84.237)	236.00 (41.981)	-20.77 (77.869)	251.14 (55.284)	-17.76 (80.495)
Median	267.00	6.25	245.00	-16.00	257.00	-3.50
Min, Max	142.0, 403.0	-206.5, 78.0	162.0, 307.0	-138.0, 120.0	142.0, 403.0	-206.5, 120.0
Week 80						
n	17	17	8	8	25	25
Mean (SD)	277.06 (70.692)	-2.91 (92.079)	243.13 (72.292)	-13.13 (39.669)	266.20 (71.532)	-6.18 (78.326)
Median	279.00	-6.00	240.50	-15.00	272.00	-12.50
Min, Max	153.0, 449.0	-162.5, 215.5	171.0, 389.0	-72.5, 52.0	153.0, 449.0	-162.5, 215.5

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.8

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	11	11	7	7	18	18
Mean (SD)	305.55 (93.025)	46.32 (106.489)	242.29 (28.022)	5.36 (50.576)	280.94 (79.841)	30.39 (89.418)
Median	292.00	21.50	246.00	9.00	267.00	16.00
Min, Max	176.0, 529.0	-91.0, 329.0	211.0, 274.0	-93.5, 59.0	176.0, 529.0	-93.5, 329.0
Week 96						
n	7	7	6	6	13	13
Mean (SD)	250.29 (58.100)	-18.86 (53.427)	242.67 (64.590)	-3.75 (51.607)	246.77 (58.666)	-11.88 (50.974)
Median	246.00	-10.50	228.50	-24.75	230.00	-17.50
Min, Max	165.0, 348.0	-105.0, 61.5	178.0, 342.0	-52.5, 77.0	165.0, 348.0	-105.0, 77.0
Week 104						
n	1	1	2	2	3	3
Mean (SD)	193.00 (NA)	-44.50 (NA)	256.00 (49.497)	-21.25 (78.135)	235.00 (50.478)	-29.00 (56.857)
Median	193.00	-44.50	256.00	-21.25	221.00	-44.50
Min, Max	193.0, 193.0	-44.5, -44.5	221.0, 291.0	-76.5, 34.0	193.0, 291.0	-76.5, 34.0

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	305.55 (87.827)		252.44 (87.624)		290.13 (89.707)	
Median	291.00		230.50		261.50	
Min, Max	172.0, 476.0		139.0, 404.0		139.0, 476.0	
Week 1						
n	21	21	10	9		
Mean (SD)	253.43 (63.679)	-54.67 (46.831)	338.40 (330.264)	-16.44 (23.324)		
Median	249.00	-42.00	233.00	-11.00		
Min, Max	137.0, 396.0	-169.0, 9.5	128.0, 1260.0	-60.0, 13.0		
Week 2						
n	22	22	10	9		
Mean (SD)	251.32 (55.852)	-54.23 (64.507)	339.00 (331.069)	-15.78 (31.402)		
Median	260.50	-44.50	226.00	0.50		
Min, Max	128.0, 354.0	-199.0, 66.5	144.0, 1260.0	-73.0, 5.5		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	22	22	10	9		
Mean (SD)	248.45 (59.978)	-57.09 (64.982)	332.30 (332.505)	-23.22 (29.974)		
Median	240.50	-42.00	225.00	-10.50		
Min, Max	132.0, 370.0	-263.0, 24.0	123.0, 1260.0	-83.0, 2.5		
Week 4						
n	21	21	10	9		
Mean (SD)	255.52 (56.947)	-48.60 (73.029)	349.40 (328.114)	-4.22 (19.431)		
Median	261.00	-40.00	256.50	-5.00		
Min, Max	147.0, 341.0	-209.0, 114.0	123.0, 1260.0	-29.0, 17.5		
Week 6						
n	22	22	10	9		
Mean (SD)	266.82 (48.582)	-38.73 (73.114)	364.20 (334.247)	12.22 (41.870)		
Median	261.00	-6.50	235.50	1.50		
Min, Max	191.0, 359.0	-201.0, 78.0	130.0, 1260.0	-46.5, 98.0		

Baseline is defined as the average of all available assessments prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB
Run Date: 2024-04-26T15:50:36

Data cutoff date of 31Mar2023
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	21	21	10	9		
Mean (SD)	277.52 (66.534)	-24.50 (52.251)	372.30 (328.675)	21.22 (34.268)		
Median	257.00	-6.00	253.50	17.50		
Min, Max	183.0, 394.0	-160.0, 35.5	122.0, 1260.0	-17.0, 92.5		
Week 10						
n	21	21	10	9		
Mean (SD)	278.90 (54.471)	-28.74 (71.985)	348.60 (329.940)	-5.11 (27.463)		
Median	270.00	-18.00	226.50	9.00		
Min, Max	204.0, 365.0	-213.0, 55.0	155.0, 1260.0	-67.5, 16.0		
Week 12						
n	22	22	10	9		
Mean (SD)	278.45 (59.536)	-27.09 (67.730)	351.00 (329.647)	-2.44 (24.493)		
Median	273.50	-13.25	241.50	-5.00		
Min, Max	194.0, 453.0	-200.0, 53.0	128.0, 1260.0	-54.5, 31.0		

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	22	22	9	8		
Mean (SD)	290.50 (140.640)	-15.05 (160.693)	282.67 (222.046)	-26.88 (47.033)		
Median	266.50	-5.50	219.00	-13.00		
Min, Max	182.0, 882.0	-223.0, 627.5	122.0, 854.0	-128.0, 27.5		
Week 14						
n	22	22	9	8		
Mean (SD)	288.18 (93.703)	-17.36 (88.933)	250.67 (114.096)	-18.75 (36.681)		
Median	273.50	-17.25	224.00	-10.75		
Min, Max	152.0, 573.0	-187.0, 205.5	118.0, 501.0	-101.0, 22.5		
Week 15						
n	21	21	9	8		
Mean (SD)	267.76 (54.805)	-41.62 (66.847)	222.67 (70.139)	-32.75 (45.705)		
Median	263.00	-21.00	221.00	-18.25		
Min, Max	174.0, 418.0	-198.0, 23.5	123.0, 361.0	-127.0, 11.5		

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	21	21	9	8		
Mean (SD)	304.24 (102.304)	-0.52 (104.935)	239.89 (80.971)	-8.00 (30.074)		
Median	302.00	-6.00	245.00	-9.25		
Min, Max	180.0, 639.0	-146.0, 332.0	121.0, 390.0	-65.5, 26.0		
Week 18						
n	22	22	8	7		
Mean (SD)	268.50 (54.092)	-37.05 (66.056)	245.63 (63.361)	5.50 (56.811)		
Median	267.50	-5.50	268.00	17.00		
Min, Max	190.0, 374.0	-191.0, 33.0	141.0, 309.0	-105.0, 67.5		
Week 20						
n	22	22	9	8		
Mean (SD)	271.41 (63.259)	-34.14 (54.840)	251.00 (70.727)	0.75 (61.557)		
Median	264.00	-28.00	269.00	3.25		
Min, Max	191.0, 434.0	-159.0, 38.5	137.0, 348.0	-127.0, 84.5		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	21	21	9	8		
Mean (SD)	269.19 (60.159)	-42.71 (70.624)	257.78 (58.465)	10.38 (82.237)		
Median	275.00	-16.00	256.00	33.50		
Min, Max	175.0, 363.0	-192.0, 55.5	169.0, 332.0	-180.0, 83.5		
Week 24						
n	20	20	8	7		
Mean (SD)	295.50 (79.694)	-20.75 (88.406)	289.88 (71.989)	29.14 (78.266)		
Median	292.00	-14.50	307.50	45.00		
Min, Max	187.0, 490.0	-175.0, 142.0	183.0, 366.0	-116.0, 135.5		
Week 28						
n	22	22	8	7	30	29
Mean (SD)	292.05 (97.220)	-13.50 (110.236)	288.75 (55.987)	29.86 (80.764)	291.17 (87.196)	-3.03 (104.251)
Median	271.00	-9.00	305.00	22.50	276.00	-7.00
Min, Max	203.0, 632.0	-208.0, 370.5	195.0, 342.0	-104.0, 143.5	195.0, 632.0	-208.0, 370.5

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Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	21	21	7	6	28	27
Mean (SD)	290.14 (76.550)	-14.62 (62.442)	280.43 (86.506)	11.58 (91.341)	287.71 (77.601)	-8.80 (68.753)
Median	275.00	-4.00	267.00	21.25	272.00	0.00
Min, Max	189.0, 467.0	-185.0, 79.5	154.0, 421.0	-137.0, 133.5	154.0, 467.0	-185.0, 133.5
Week 36						
n	21	21	7	6	28	27
Mean (SD)	309.43 (131.963)	1.33 (128.230)	271.57 (66.903)	6.25 (44.340)	299.96 (119.049)	2.43 (114.152)
Median	285.00	-5.00	282.00	-3.75	283.50	-5.00
Min, Max	193.0, 804.0	-172.0, 470.0	166.0, 352.0	-52.0, 60.0	166.0, 804.0	-172.0, 470.0
Week 40						
n	20	20	7	6	27	26
Mean (SD)	315.00 (108.096)	1.70 (98.449)	258.57 (60.268)	-13.75 (78.290)	300.37 (100.061)	-1.87 (92.930)
Median	284.50	-0.75	265.00	6.25	278.00	0.00
Min, Max	201.0, 623.0	-193.0, 209.0	179.0, 357.0	-164.0, 46.0	179.0, 623.0	-193.0, 209.0

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Post-hoc Analysen Studie ALPHA

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Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	19	19	6	5	25	24
Mean (SD)	283.74 (52.602)	-24.58 (56.968)	263.33 (51.200)	-19.40 (91.963)	278.84 (51.966)	-23.50 (63.366)
Median	292.00	-19.50	261.00	17.00	289.00	-19.00
Min, Max	183.0, 394.0	-148.0, 93.0	197.0, 328.0	-180.0, 50.5	183.0, 394.0	-180.0, 93.0
Week 48						
n	17	17	6	5	23	22
Mean (SD)	305.59 (78.100)	-9.44 (87.978)	310.50 (76.189)	31.80 (79.761)	306.87 (75.896)	-0.07 (86.151)
Median	302.00	-17.50	325.50	31.00	304.00	-12.50
Min, Max	173.0, 455.0	-157.0, 230.0	211.0, 413.0	-88.0, 125.5	173.0, 455.0	-157.0, 230.0
Week 52						
n	17	17	5	4	22	21
Mean (SD)	286.65 (60.878)	-20.94 (65.600)	282.60 (65.634)	-11.38 (84.903)	285.73 (60.393)	-19.12 (67.371)
Median	299.00	-10.50	266.00	27.50	288.50	8.50
Min, Max	186.0, 387.0	-154.0, 67.0	198.0, 368.0	-138.0, 37.5	186.0, 387.0	-154.0, 67.0

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	16	16	5	4	21	20
Mean (SD)	293.19 (55.449)	-21.41 (67.715)	283.60 (64.736)	-13.38 (90.654)	290.90 (56.228)	-19.80 (70.203)
Median	285.00	0.25	255.00	27.25	283.00	2.50
Min, Max	197.0, 392.0	-154.0, 85.0	221.0, 381.0	-149.0, 41.0	197.0, 392.0	-154.0, 85.0
Week 60						
n	16	16	5	4	21	20
Mean (SD)	282.19 (72.170)	-32.41 (90.354)	281.60 (66.406)	-14.63 (91.051)	282.05 (69.199)	-28.85 (88.359)
Median	270.50	-39.00	253.00	28.50	261.00	-30.50
Min, Max	181.0, 444.0	-167.0, 204.0	213.0, 376.0	-151.0, 35.5	181.0, 444.0	-167.0, 204.0
Week 64						
n	14	14	5	4	19	18
Mean (SD)	290.79 (59.015)	-33.04 (63.136)	313.00 (64.911)	21.13 (59.049)	296.63 (59.604)	-21.00 (64.810)
Median	313.00	-19.75	337.00	47.00	319.00	-10.75
Min, Max	186.0, 366.0	-166.0, 66.5	231.0, 390.0	-67.0, 57.5	186.0, 390.0	-166.0, 66.5

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Modified Randomized Set

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	11	11	4	4	15	15
Mean (SD)	281.73 (61.130)	-26.55 (68.577)	265.75 (61.846)	-6.88 (76.881)	277.47 (59.517)	-21.30 (68.606)
Median	284.00	-14.00	259.00	18.00	284.00	3.50
Min, Max	176.0, 412.0	-192.0, 41.5	202.0, 343.0	-119.0, 55.5	176.0, 412.0	-192.0, 55.5
Week 72						
n	9	9	3	3	12	12
Mean (SD)	270.44 (64.707)	-28.61 (89.968)	238.33 (37.233)	-29.33 (94.537)	262.42 (59.229)	-28.79 (86.670)
Median	269.00	-3.50	253.00	16.00	261.50	6.25
Min, Max	191.0, 403.0	-193.0, 78.0	196.0, 266.0	-138.0, 34.0	191.0, 403.0	-193.0, 78.0
Week 80						
n	8	8	3	3	11	11
Mean (SD)	279.50 (48.853)	-25.81 (70.201)	270.33 (107.937)	2.67 (20.404)	277.00 (63.396)	-18.05 (60.910)
Median	276.50	-13.00	244.00	-2.00	274.00	-2.00
Min, Max	214.0, 355.0	-147.0, 42.0	178.0, 389.0	-15.0, 25.0	178.0, 389.0	-147.0, 42.0

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:36

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	4	4	2	2	6	6
Mean (SD)	287.00 (54.979)	-15.00 (55.927)	237.50 (36.062)	38.00 (8.485)	270.50 (52.222)	2.67 (51.383)
Median	272.00	-4.25	237.50	38.00	256.50	21.00
Min, Max	242.0, 362.0	-91.0, 39.5	212.0, 263.0	32.0, 44.0	212.0, 362.0	-91.0, 44.0
Week 96						
n	3	3	1	1	4	4
Mean (SD)	281.33 (57.770)	-36.50 (59.894)	296.00 (NA)	77.00 (NA)	285.00 (47.735)	-8.13 (74.914)
Median	250.00	-10.50	296.00	77.00	273.00	-2.25
Min, Max	246.0, 348.0	-105.0, 6.0	296.0, 296.0	77.0, 77.0	246.0, 348.0	-105.0, 77.0

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		11		39	
Mean (SD)	297.07 (149.558)		306.50 (86.641)		299.73 (133.741)	
Median	244.00		294.00		254.50	
Min, Max	140.0, 809.0		202.0, 506.0		140.0, 809.0	
Week 1						
n	24	24	11	11		
Mean (SD)	235.83 (90.994)	-64.45 (83.878)	283.00 (83.644)	-23.50 (119.229)		
Median	220.00	-46.00	252.00	-13.00		
Min, Max	147.0, 532.0	-295.5, 87.0	174.0, 404.0	-332.0, 155.0		
Week 2						
n	28	28	11	11		
Mean (SD)	228.18 (91.715)	-68.89 (79.850)	345.73 (289.314)	39.23 (322.947)		
Median	211.00	-50.00	264.00	-12.00		
Min, Max	117.0, 515.0	-294.0, 20.5	161.0, 1195.0	-345.0, 955.0		

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	25	25	10	10		
Mean (SD)	220.40 (88.847)	-80.26 (89.925)	277.20 (85.973)	-9.35 (96.035)		
Median	201.00	-54.00	262.50	-6.75		
Min, Max	121.0, 540.0	-398.5, 9.5	149.0, 433.0	-185.0, 193.0		
Week 4						
n	28	28	7	7		
Mean (SD)	251.71 (104.057)	-45.35 (84.341)	268.29 (53.344)	-6.57 (51.365)		
Median	223.50	-16.75	255.00	-15.00		
Min, Max	108.0, 551.0	-259.5, 98.5	178.0, 344.0	-82.5, 76.5		
Week 6						
n	25	25	10	10		
Mean (SD)	279.76 (155.653)	-20.75 (61.878)	326.80 (194.410)	40.25 (211.718)		
Median	248.00	-25.50	257.50	-15.00		
Min, Max	124.0, 794.0	-116.5, 142.0	167.0, 843.0	-167.0, 603.0		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	26	26	10	10		
Mean (SD)	288.23 (128.153)	-12.76 (112.534)	322.40 (137.818)	35.85 (150.474)		
Median	251.50	-18.00	283.00	-5.00		
Min, Max	145.0, 629.0	-337.5, 255.5	180.0, 672.0	-113.5, 432.0		
Week 10						
n	23	23	10	10		
Mean (SD)	256.26 (92.658)	-12.62 (59.050)	294.40 (95.167)	7.85 (107.702)		
Median	231.00	-12.00	261.50	-12.75		
Min, Max	148.0, 589.0	-220.0, 82.5	168.0, 459.0	-137.0, 191.5		
Week 12						
n	23	23	10	10		
Mean (SD)	256.83 (86.812)	-31.28 (108.976)	285.00 (62.534)	-1.55 (48.800)		
Median	249.00	-10.00	280.50	8.00		
Min, Max	135.0, 519.0	-379.5, 99.5	179.0, 394.0	-84.5, 64.5		

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Post-hoc Analysen Studie ALPHA

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White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	24	24	9	9		
Mean (SD)	264.75 (113.653)	-29.35 (117.607)	212.44 (40.125)	-66.33 (70.413)		
Median	244.50	-13.75	224.00	-64.50		
Min, Max	129.0, 639.0	-496.5, 133.5	138.0, 259.0	-196.0, 12.0		
Week 14						
n	24	24	9	9		
Mean (SD)	269.42 (107.070)	-21.58 (130.076)	222.11 (24.385)	-59.17 (63.848)		
Median	262.00	-11.00	221.00	-67.50		
Min, Max	135.0, 587.0	-442.5, 214.5	190.0, 256.0	-155.5, 7.0		
Week 15						
n	24	24	9	9		
Mean (SD)	272.00 (122.710)	-19.13 (142.520)	218.33 (40.370)	-70.33 (68.557)		
Median	245.50	-8.75	210.00	-55.00		
Min, Max	134.0, 627.0	-486.5, 283.5	166.0, 279.0	-168.0, 29.0		

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	26	26	10	10		
Mean (SD)	278.35 (116.139)	-12.07 (102.534)	228.10 (44.488)	-58.45 (70.590)		
Median	233.00	-10.25	236.00	-64.75		
Min, Max	144.0, 582.0	-258.5, 243.5	152.0, 297.0	-182.0, 50.0		
Week 18						
n	25	25	10	10		
Mean (SD)	269.08 (101.584)	-18.87 (94.822)	268.60 (79.273)	-17.95 (68.845)		
Median	260.00	-2.50	254.50	-29.75		
Min, Max	159.0, 568.0	-297.5, 113.5	200.0, 461.0	-111.0, 93.5		
Week 20						
n	21	21	10	10		
Mean (SD)	297.19 (153.747)	-5.25 (89.306)	253.80 (47.988)	-32.75 (55.286)		
Median	255.00	-14.50	250.00	-21.25		
Min, Max	157.0, 725.0	-228.5, 235.5	185.0, 325.0	-109.0, 71.0		

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White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	26	26	10	10		
Mean (SD)	268.58 (154.010)	-21.84 (85.796)	270.90 (50.538)	-15.65 (66.420)		
Median	242.00	-19.25	289.00	-13.75		
Min, Max	108.0, 906.0	-307.0, 171.5	184.0, 349.0	-122.5, 67.0		
Week 24						
n	25	25	10	10		
Mean (SD)	251.64 (88.760)	-40.21 (123.207)	266.00 (61.624)	-20.55 (74.887)		
Median	243.00	-18.00	254.00	-27.75		
Min, Max	130.0, 506.0	-491.5, 116.0	188.0, 394.0	-125.0, 73.0		
Week 28						
n	24	24	10	10	34	34
Mean (SD)	249.13 (91.902)	-42.43 (114.200)	269.70 (72.068)	-16.85 (88.094)	255.18 (85.986)	-34.91 (106.518)
Median	240.00	-28.00	272.50	7.00	244.00	-21.25
Min, Max	134.0, 591.0	-467.5, 101.5	173.0, 405.0	-161.0, 100.0	134.0, 591.0	-467.5, 101.5

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Post-hoc Analysen Studie ALPHA

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White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	20	20	10	10	30	30
Mean (SD)	266.50 (97.341)	-34.50 (106.107)	283.40 (90.505)	-3.15 (108.109)	272.13 (93.892)	-24.05 (105.969)
Median	244.00	-8.75	256.50	1.00	249.50	-6.50
Min, Max	150.0, 512.0	-331.5, 101.0	191.0, 421.0	-142.0, 153.5	150.0, 512.0	-331.5, 153.5
Week 36						
n	20	20	8	8	28	28
Mean (SD)	239.60 (52.194)	-15.37 (55.911)	276.50 (81.230)	6.00 (63.641)	250.14 (62.577)	-9.26 (57.849)
Median	254.00	-20.50	255.00	-3.50	254.00	-14.75
Min, Max	131.0, 318.0	-103.5, 111.7	172.0, 426.0	-62.5, 107.0	131.0, 426.0	-103.5, 111.7
Week 40						
n	19	19	9	9	28	28
Mean (SD)	251.32 (48.891)	-25.89 (99.090)	272.78 (77.810)	-18.17 (70.028)	258.21 (59.090)	-23.40 (89.512)
Median	253.00	-0.50	271.00	-21.50	255.00	-13.25
Min, Max	170.0, 339.0	-395.5, 54.5	170.0, 376.0	-117.0, 120.0	170.0, 376.0	-395.5, 120.0

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

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White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	17	17	10	10	27	27
Mean (SD)	239.53 (56.321)	-41.34 (115.279)	264.70 (65.310)	-21.85 (78.949)	248.85 (59.849)	-34.12 (102.116)
Median	243.00	-27.00	271.50	-29.25	252.00	-27.00
Min, Max	149.0, 318.0	-443.5, 78.0	164.0, 373.0	-118.0, 81.0	149.0, 373.0	-443.5, 81.0
Week 48						
n	14	14	9	9	23	23
Mean (SD)	260.57 (64.821)	4.21 (65.263)	253.44 (70.592)	-27.83 (65.845)	257.78 (65.633)	-8.33 (65.948)
Median	251.00	2.75	276.00	-16.50	252.00	-8.50
Min, Max	157.0, 405.0	-115.5, 126.5	170.0, 351.0	-164.0, 41.0	157.0, 405.0	-164.0, 126.5
Week 52						
n	14	14	9	9	23	23
Mean (SD)	247.36 (57.819)	-44.50 (119.181)	257.89 (49.387)	-23.39 (71.946)	251.48 (53.759)	-36.24 (101.915)
Median	238.00	-34.50	247.00	-36.50	242.00	-34.50
Min, Max	155.0, 334.0	-436.5, 82.5	175.0, 347.0	-125.5, 100.0	155.0, 347.0	-436.5, 100.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	15	15	8	8	23	23
Mean (SD)	285.47 (89.340)	-2.77 (120.425)	319.50 (157.067)	33.94 (143.706)	297.30 (114.906)	10.00 (126.961)
Median	272.00	-12.50	278.50	-17.50	272.00	-16.00
Min, Max	153.0, 439.0	-329.5, 225.0	186.0, 673.0	-87.5, 339.0	153.0, 673.0	-329.5, 339.0
Week 60						
n	12	12	7	7	19	19
Mean (SD)	252.67 (52.295)	0.46 (53.520)	278.57 (97.158)	-13.50 (85.344)	262.21 (70.588)	-4.68 (65.009)
Median	261.50	7.00	289.00	-36.00	275.00	-4.50
Min, Max	161.0, 313.0	-103.5, 75.5	166.0, 444.0	-119.0, 110.0	161.0, 444.0	-119.0, 110.0
Week 64						
n	11	11	6	6	17	17
Mean (SD)	268.73 (58.226)	15.27 (51.049)	241.67 (91.121)	-39.67 (76.072)	259.18 (69.938)	-4.12 (64.572)
Median	289.00	28.50	216.50	-39.25	242.00	-14.50
Min, Max	151.0, 334.0	-61.5, 76.5	167.0, 421.0	-131.5, 87.0	151.0, 421.0	-131.5, 87.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:36

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	9	9	5	5	14	14
Mean (SD)	269.22 (52.311)	34.22 (49.591)	207.00 (44.079)	-63.80 (71.046)	247.00 (56.913)	-0.79 (73.771)
Median	278.00	46.50	198.00	-69.50	270.00	10.00
Min, Max	143.0, 333.0	-44.0, 99.5	162.0, 267.0	-131.5, 44.0	143.0, 333.0	-131.5, 99.5
Week 72						
n	7	7	5	5	12	12
Mean (SD)	247.71 (69.871)	16.29 (43.727)	222.20 (46.874)	-48.60 (67.930)	237.08 (60.286)	-10.75 (61.946)
Median	266.00	22.00	245.00	-22.50	251.50	-21.25
Min, Max	142.0, 340.0	-30.0, 69.5	162.0, 264.0	-132.0, 35.0	142.0, 340.0	-132.0, 69.5
Week 80						
n	6	6	4	4	10	10
Mean (SD)	292.50 (98.254)	58.17 (98.670)	223.75 (56.400)	-41.25 (26.008)	265.00 (87.658)	18.40 (90.939)
Median	299.50	43.00	214.50	-38.75	268.50	-13.75
Min, Max	153.0, 449.0	-60.0, 215.5	171.0, 295.0	-72.5, -15.0	153.0, 449.0	-72.5, 215.5

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	3	3	9	9
Mean (SD)	329.00 (116.931)	94.67 (120.782)	252.00 (35.539)	-27.00 (57.656)	303.33 (101.703)	54.11 (116.831)
Median	321.00	45.25	271.00	3.50	277.00	21.50
Min, Max	176.0, 529.0	10.5, 329.0	211.0, 274.0	-93.5, 9.0	176.0, 529.0	-93.5, 329.0
Week 96						
n	3	3	3	3	6	6
Mean (SD)	229.33 (65.010)	-1.67 (63.758)	249.00 (84.184)	-30.00 (9.124)	239.17 (68.128)	-15.83 (43.591)
Median	228.00	-0.50	227.00	-25.50	227.50	-24.75
Min, Max	165.0, 295.0	-66.0, 61.5	178.0, 342.0	-40.5, -24.0	165.0, 342.0	-66.0, 61.5
Week 104						
n	0	0	1	1	1	1
Mean (SD)			291.00 (NA)	-76.50 (NA)	291.00 (NA)	-76.50 (NA)
Median			291.00	-76.50	291.00	-76.50
Min, Max			291.0, 291.0	-76.5, -76.5	291.0, 291.0	-76.5, -76.5

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	6		5		11	
Mean (SD)	330.67 (117.175)		269.10 (71.415)		302.68 (99.694)	
Median	304.75		245.00		296.00	
Min, Max	194.0, 479.5		187.0, 372.0		187.0, 479.5	
Week 1						
n	6	5	4	4		
Mean (SD)	268.67 (100.876)	-118.30 (96.494)	258.25 (18.025)	-19.25 (66.535)		
Median	243.50	-94.00	253.50	-22.00		
Min, Max	178.0, 457.0	-243.5, -16.0	243.0, 283.0	-89.0, 56.0		
Week 2						
n	7	6	5	5		
Mean (SD)	317.71 (284.361)	-118.83 (113.648)	271.60 (50.535)	2.50 (46.282)		
Median	213.00	-75.25	262.00	-17.00		
Min, Max	156.0, 953.0	-307.5, -15.0	217.0, 355.0	-44.0, 76.0		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	7	6	4	4		
Mean (SD)	218.14 (68.356)	-133.17 (105.789)	294.50 (94.620)	17.00 (115.796)		
Median	230.00	-81.50	282.00	-3.50		
Min, Max	154.0, 342.0	-301.5, -40.0	193.0, 421.0	-101.0, 176.0		
Week 4						
n	7	6	5	5		
Mean (SD)	342.71 (238.737)	-72.00 (81.674)	296.00 (70.806)	26.90 (18.750)		
Median	255.00	-56.00	294.00	22.00		
Min, Max	159.0, 847.0	-224.5, -3.0	197.0, 389.0	10.0, 58.5		
Week 6						
n	7	6	5	5		
Mean (SD)	269.43 (125.042)	-104.50 (87.216)	276.00 (21.852)	6.90 (87.370)		
Median	225.00	-79.75	265.00	22.50		
Min, Max	146.0, 529.0	-238.5, -26.5	258.0, 312.0	-108.0, 125.0		

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Post-hoc Analysen Studie ALPHA

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ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	7	6	4	4		
Mean (SD)	286.00 (111.574)	-65.00 (24.876)	269.75 (13.817)	26.38 (37.526)		
Median	257.00	-57.00	273.00	32.75		
Min, Max	146.0, 412.0	-104.5, -39.0	251.0, 282.0	-24.0, 64.0		
Week 10						
n	7	6	5	5		
Mean (SD)	237.86 (48.831)	-101.83 (80.151)	255.80 (35.773)	-13.30 (59.420)		
Median	263.00	-73.50	259.00	5.00		
Min, Max	165.0, 292.0	-202.5, -29.0	218.0, 311.0	-112.0, 44.0		
Week 12						
n	7	6	5	5		
Mean (SD)	252.57 (40.820)	-79.67 (112.065)	278.40 (77.555)	9.30 (45.323)		
Median	262.00	-47.75	255.00	10.00		
Min, Max	171.0, 301.0	-230.5, 63.5	199.0, 386.0	-43.0, 80.0		

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Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	7	6	5	5		
Mean (SD)	238.86 (41.018)	-91.83 (110.820)	207.20 (36.321)	-61.90 (64.084)		
Median	239.00	-55.00	219.00	-42.00		
Min, Max	194.0, 299.0	-259.5, 11.0	145.0, 239.0	-162.0, 3.5		
Week 14						
n	7	6	5	5		
Mean (SD)	238.71 (31.293)	-96.83 (105.916)	226.00 (57.110)	-43.10 (82.908)		
Median	238.00	-54.50	220.00	-12.50		
Min, Max	195.0, 281.0	-243.5, 1.0	182.0, 322.0	-189.0, 16.0		
Week 15						
n	6	5	4	4		
Mean (SD)	258.00 (76.034)	-91.60 (89.846)	215.50 (42.775)	-27.88 (31.012)		
Median	241.00	-67.50	230.50	-29.50		
Min, Max	184.0, 377.0	-243.5, -7.0	154.0, 247.0	-64.0, 11.5		

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Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	7	6	5	5		
Mean (SD)	252.43 (59.565)	-85.67 (115.509)	293.60 (87.446)	24.50 (140.016)		
Median	247.00	-39.50	255.00	4.00		
Min, Max	165.0, 346.0	-239.5, 50.0	225.0, 439.0	-133.0, 252.0		
Week 18						
n	7	6	5	5		
Mean (SD)	254.29 (68.332)	-82.17 (120.070)	267.00 (78.502)	-2.10 (29.198)		
Median	252.00	-70.00	239.00	-12.00		
Min, Max	187.0, 384.0	-222.5, 88.0	175.0, 352.0	-26.0, 46.0		
Week 20						
n	6	5	5	5		
Mean (SD)	267.67 (38.281)	-97.80 (127.797)	317.60 (130.770)	48.50 (102.970)		
Median	259.00	-71.50	250.00	14.50		
Min, Max	220.0, 321.0	-243.5, 25.0	213.0, 535.0	-29.0, 229.0		

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Post-hoc Analysen Studie ALPHA

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Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	7	6	5	5		
Mean (SD)	260.71 (65.853)	-86.17 (86.239)	269.00 (84.758)	-0.10 (91.792)		
Median	261.00	-61.00	236.00	17.00		
Min, Max	168.0, 358.0	-224.5, -4.0	204.0, 416.0	-145.0, 110.0		
Week 24						
n	7	6	5	5		
Mean (SD)	310.14 (123.900)	-23.17 (98.411)	295.20 (86.768)	26.10 (96.439)		
Median	277.00	-16.50	247.00	51.00		
Min, Max	180.0, 573.0	-201.5, 93.5	238.0, 442.0	-125.0, 136.0		
Week 28						
n	5	5	5	5	10	10
Mean (SD)	293.60 (80.559)	-44.00 (116.817)	282.00 (86.848)	12.90 (85.741)	287.80 (79.208)	-15.55 (101.152)
Median	308.00	-64.50	251.00	-13.00	270.50	-14.25
Min, Max	173.0, 389.0	-196.5, 114.0	220.0, 433.0	-98.0, 127.0	173.0, 433.0	-196.5, 127.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:36

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	7	6	5	5	12	11
Mean (SD)	275.57 (97.279)	-89.83 (117.103)	243.80 (39.360)	-25.30 (81.948)	262.33 (77.413)	-60.50 (103.337)
Median	250.00	-44.75	227.00	-1.00	250.00	-18.00
Min, Max	179.0, 484.0	-255.5, 34.5	205.0, 305.0	-167.0, 36.0	179.0, 484.0	-255.5, 36.0
Week 36						
n	6	5	5	5	11	10
Mean (SD)	231.67 (32.611)	-113.20 (107.333)	305.20 (108.652)	36.10 (85.379)	265.09 (82.027)	-38.55 (120.631)
Median	241.00	-65.50	256.00	20.50	248.00	-34.50
Min, Max	185.0, 268.0	-229.5, 6.0	206.0, 478.0	-39.0, 172.0	185.0, 478.0	-229.5, 172.0
Week 40						
n	7	6	5	5	12	11
Mean (SD)	279.57 (39.690)	-56.50 (139.231)	287.60 (72.380)	18.50 (90.417)	282.92 (52.739)	-22.41 (120.403)
Median	277.00	-38.25	252.00	53.00	264.50	-8.00
Min, Max	239.0, 347.0	-230.5, 153.0	222.0, 394.0	-120.0, 94.5	222.0, 394.0	-230.5, 153.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:36

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	7	6	4	4	11	10
Mean (SD)	258.29 (55.380)	-76.17 (77.605)	264.75 (65.143)	21.38 (42.331)	260.64 (55.891)	-37.15 (80.501)
Median	269.00	-47.50	249.00	37.75	268.00	-27.00
Min, Max	182.0, 338.0	-210.5, -12.0	205.0, 356.0	-40.0, 50.0	182.0, 356.0	-210.5, 50.0
Week 48						
n	5	5	5	5	10	10
Mean (SD)	271.40 (62.420)	-32.70 (95.920)	265.00 (45.249)	-4.10 (65.043)	268.20 (51.508)	-18.40 (78.718)
Median	281.00	-8.50	249.00	7.50	257.00	-1.50
Min, Max	182.0, 346.0	-198.5, 50.0	226.0, 342.0	-107.0, 62.0	182.0, 346.0	-198.5, 62.0
Week 52						
n	4	4	5	5	9	9
Mean (SD)	237.00 (57.149)	-69.13 (70.346)	275.40 (104.820)	6.30 (123.182)	258.33 (84.427)	-27.22 (104.990)
Median	227.50	-48.00	241.00	30.50	241.00	-10.00
Min, Max	184.0, 309.0	-170.5, -10.0	183.0, 455.0	-189.0, 149.0	183.0, 455.0	-189.0, 149.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:36

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	5	5	5	5	10	10
Mean (SD)	340.20 (191.287)	36.10 (210.036)	283.80 (55.895)	14.70 (81.867)	312.00 (136.142)	25.40 (150.707)
Median	246.00	-3.50	256.00	47.00	251.50	28.75
Min, Max	161.0, 635.0	-233.5, 339.0	246.0, 378.0	-125.0, 72.0	161.0, 635.0	-233.5, 339.0
Week 60						
n	3	3	4	4	7	7
Mean (SD)	216.33 (55.248)	-87.33 (100.367)	265.75 (54.224)	22.38 (28.300)	244.57 (56.438)	-24.64 (84.836)
Median	188.00	-56.50	240.00	23.75	238.00	-6.00
Min, Max	181.0, 280.0	-199.5, -6.0	236.0, 347.0	-9.0, 51.0	181.0, 347.0	-199.5, 51.0
Week 64						
n	3	3	3	3	6	6
Mean (SD)	244.00 (38.626)	-59.67 (157.150)	224.67 (10.970)	2.17 (28.162)	234.33 (27.515)	-28.75 (106.502)
Median	252.00	-35.50	221.00	-8.00	229.00	-13.75
Min, Max	202.0, 278.0	-227.5, 84.0	216.0, 237.0	-19.5, 34.0	202.0, 278.0	-227.5, 84.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:36

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	3	3	3	3	6	6
Mean (SD)	232.00 (84.788)	-71.67 (101.299)	254.67 (19.858)	32.17 (49.077)	243.33 (56.458)	-19.75 (91.118)
Median	259.00	-57.00	259.00	23.50	259.00	4.75
Min, Max	137.0, 300.0	-179.5, 21.5	233.0, 272.0	-12.0, 85.0	137.0, 300.0	-179.5, 85.0
Week 72						
n	3	3	3	3	6	6
Mean (SD)	237.00 (32.450)	-66.67 (121.769)	256.67 (43.662)	34.17 (74.687)	246.83 (36.052)	-16.25 (105.889)
Median	228.00	-9.50	234.00	-1.50	231.50	-5.50
Min, Max	210.0, 273.0	-206.5, 16.0	229.0, 307.0	-16.0, 120.0	210.0, 307.0	-206.5, 120.0
Week 80						
n	3	3	1	1	4	4
Mean (SD)	239.67 (68.223)	-64.00 (85.751)	239.00 (NA)	52.00 (NA)	239.50 (55.705)	-35.00 (90.918)
Median	214.00	-23.50	239.00	52.00	226.50	-14.75
Min, Max	188.0, 317.0	-162.5, -6.0	239.0, 239.0	52.0, 52.0	188.0, 317.0	-162.5, 52.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:50:36

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.8.9

Summary of Actual Values and Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	1	1	2	2	3	3
Mean (SD)	239.00 (NA)	1.50 (NA)	232.50 (19.092)	21.25 (53.387)	234.67 (14.012)	14.67 (39.435)
Median	239.00	1.50	232.50	21.25	239.00	1.50
Min, Max	239.0, 239.0	1.5, 1.5	219.0, 246.0	-16.5, 59.0	219.0, 246.0	-16.5, 59.0
Week 96						
n	1	1	2	2	3	3
Mean (SD)	220.00 (NA)	-17.50 (NA)	206.50 (33.234)	-4.75 (67.529)	211.00 (24.759)	-9.00 (48.314)
Median	220.00	-17.50	206.50	-4.75	220.00	-17.50
Min, Max	220.0, 220.0	-17.5, -17.5	183.0, 230.0	-52.5, 43.0	183.0, 230.0	-52.5, 43.0
Week 104						
n	1	1	1	1	2	2
Mean (SD)	193.00 (NA)	-44.50 (NA)	221.00 (NA)	34.00 (NA)	207.00 (19.799)	-5.25 (55.508)
Median	193.00	-44.50	221.00	34.00	207.00	-5.25
Min, Max	193.0, 193.0	-44.5, -44.5	221.0, 221.0	34.0, 34.0	193.0, 221.0	-44.5, 34.0

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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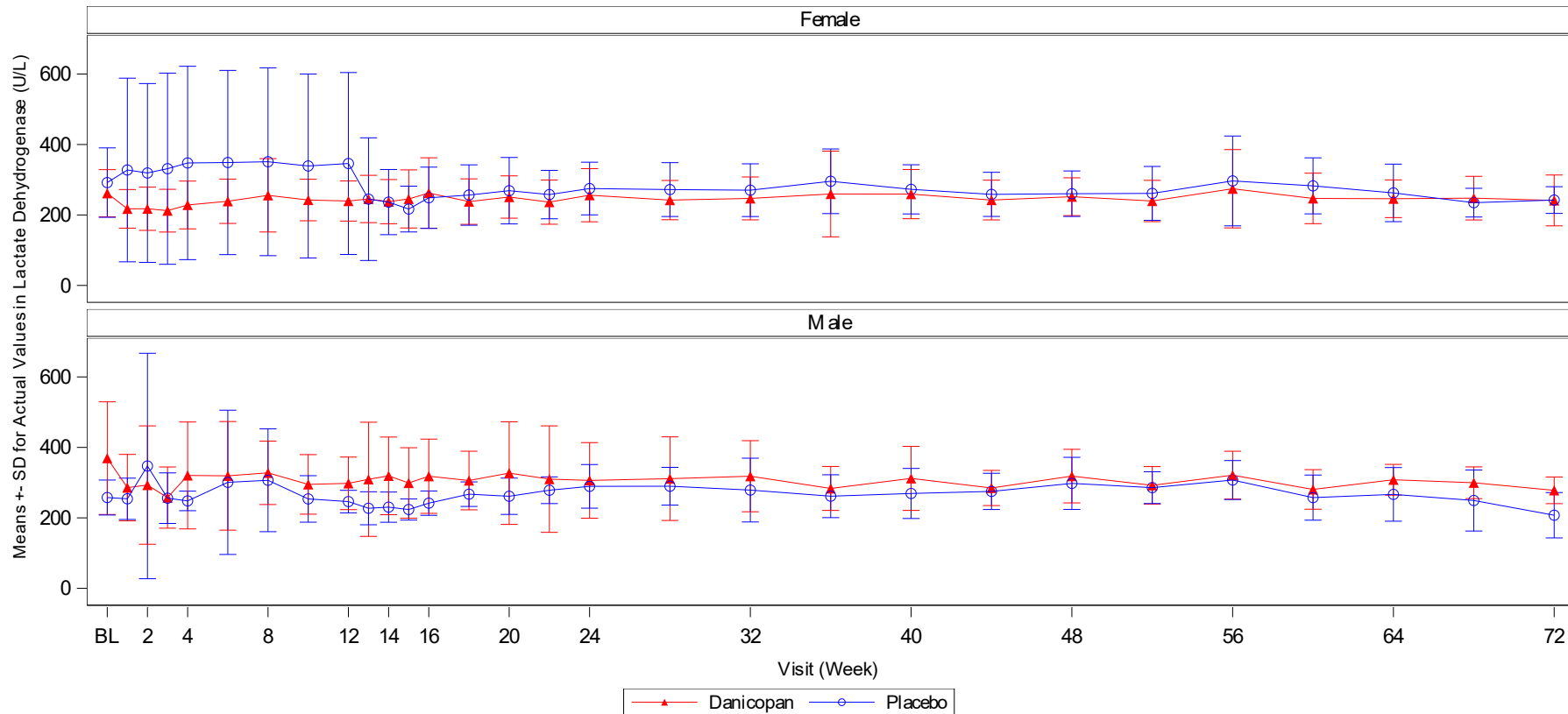
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Figure GERMANY.2.2.4.3.1
Plots of Mean \pm SD for Actual Values in Lactate Dehydrogenase (LDH) During 72 Weeks by Sex
Modified Randomized Set



Baseline is defined as the average of all available assessments prior to the first dose of study drug.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium \geq 6 mmol/L and LDH \geq 2x ULN are defined as having TTH.

Source: ADaM.ADLB

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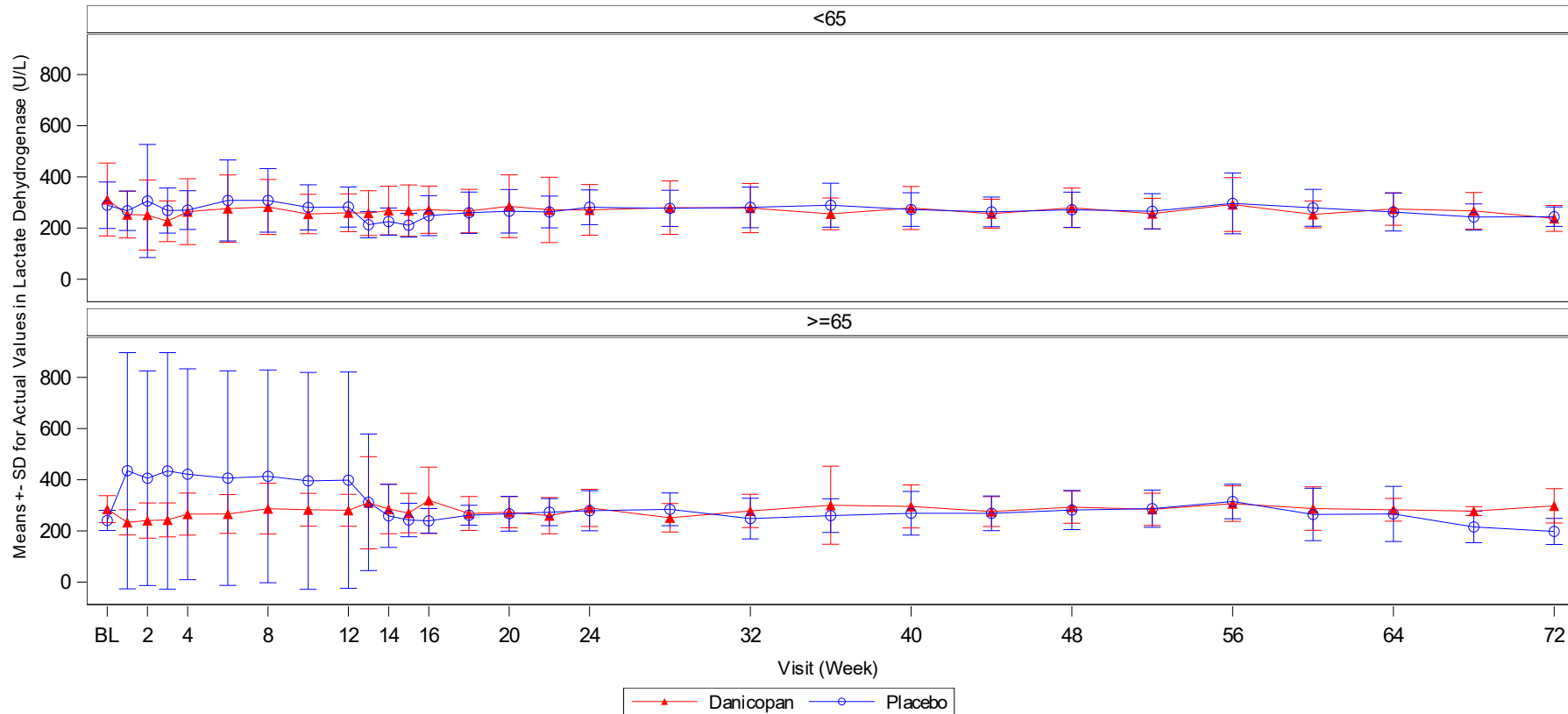
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Figure GERMANY.2.2.4.3.2
Plots of Mean \pm SD for Actual Values in Lactate Dehydrogenase (LDH) During 72 Weeks by Age at Informed Consent (< 65 and \geq 65 Years) Modified Randomized Set



Baseline is defined as the average of all available assessments prior to the first dose of study drug.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium \geq 6 mmol/L and LDH \geq 2x ULN are defined as having TTH.

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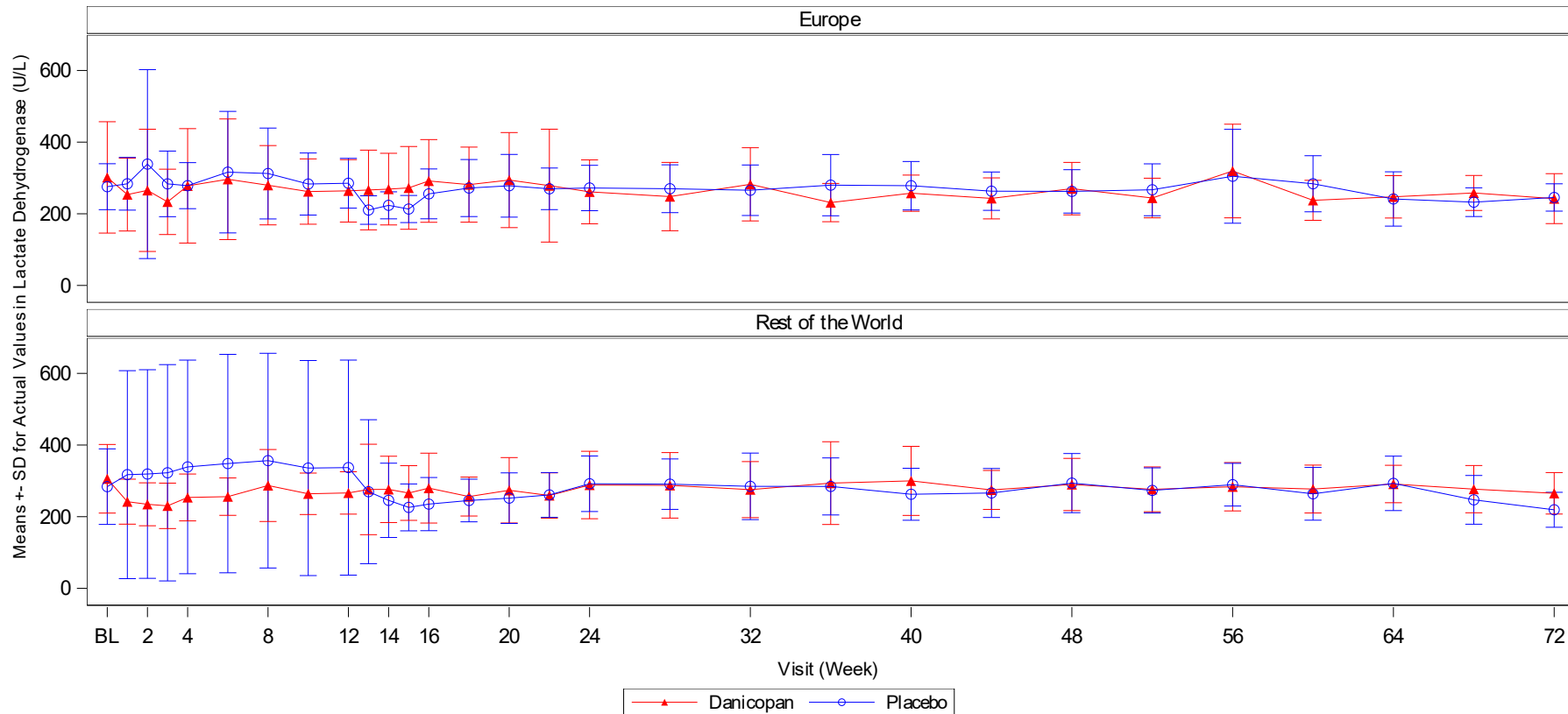
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Figure GERMANY.2.2.4.3.3
Plots of Mean \pm SD for Actual Values in Lactate Dehydrogenase (LDH) During 72 Weeks by Region
Modified Randomized Set



Baseline is defined as the average of all available assessments prior to the first dose of study drug.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.

Source: ADaM.ADLB

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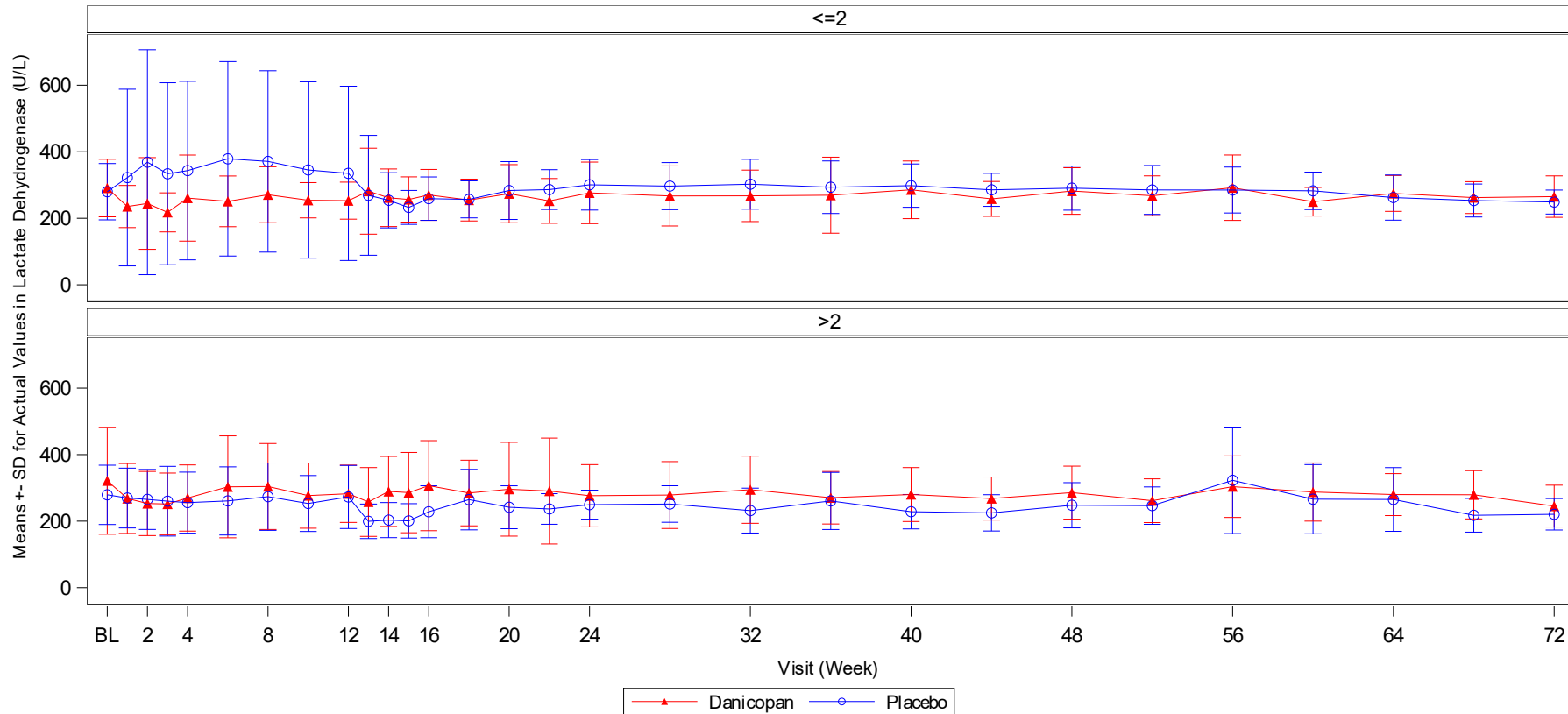
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Figure GERMANY.2.2.4.3.4

Plots of Mean \pm SD for Actual Values in Lactate Dehydrogenase (LDH) During 72 Weeks by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set



Baseline is defined as the average of all available assessments prior to the first dose of study drug.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.

Source: ADaM.ADLB

Run Date: 2024-04-26T15:31:11

Data cutoff date of 31Mar2023

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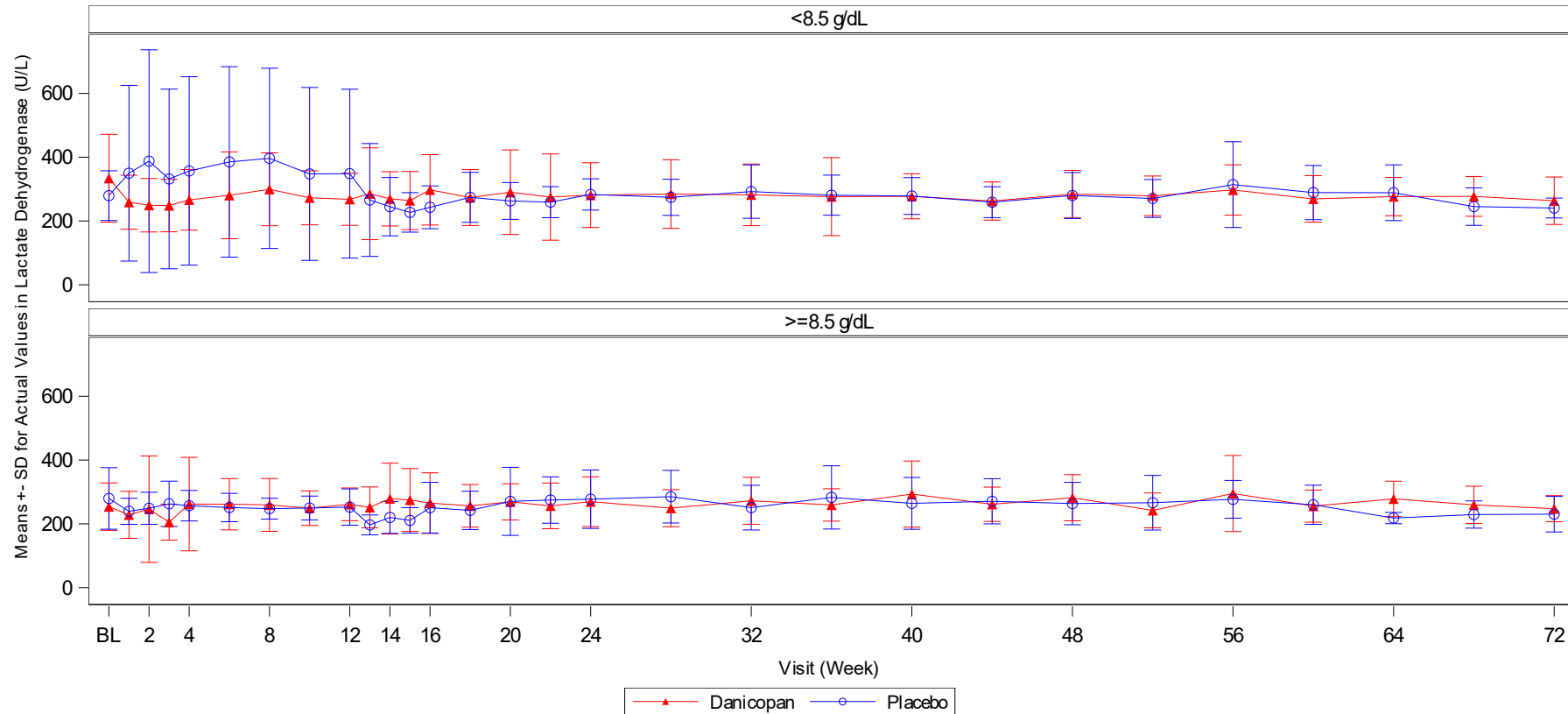
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Figure GERMANY.2.2.4.3.5

Plots of Mean \pm SD for Actual Values in Lactate Dehydrogenase (LDH) During 72 Weeks by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set



Baseline is defined as the average of all available assessments prior to the first dose of study drug.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.

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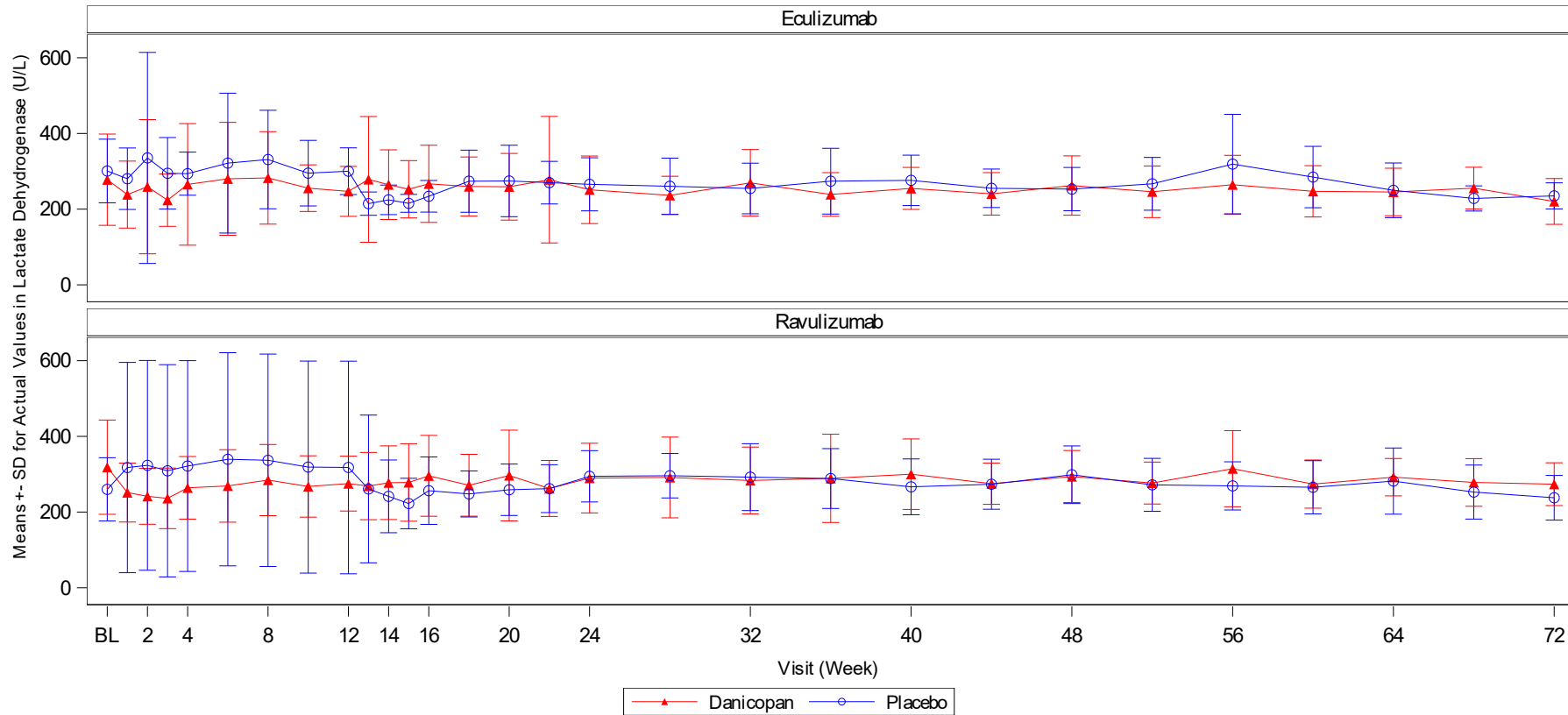
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ALXN2040-PNH-301

Figure GERMANY.2.2.4.3.6
Plots of Mean \pm SD for Actual Values in Lactate Dehydrogenase (LDH) During 72 Weeks
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set



Baseline is defined as the average of all available assessments prior to the first dose of study drug.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.

Source: ADaM.ADLB

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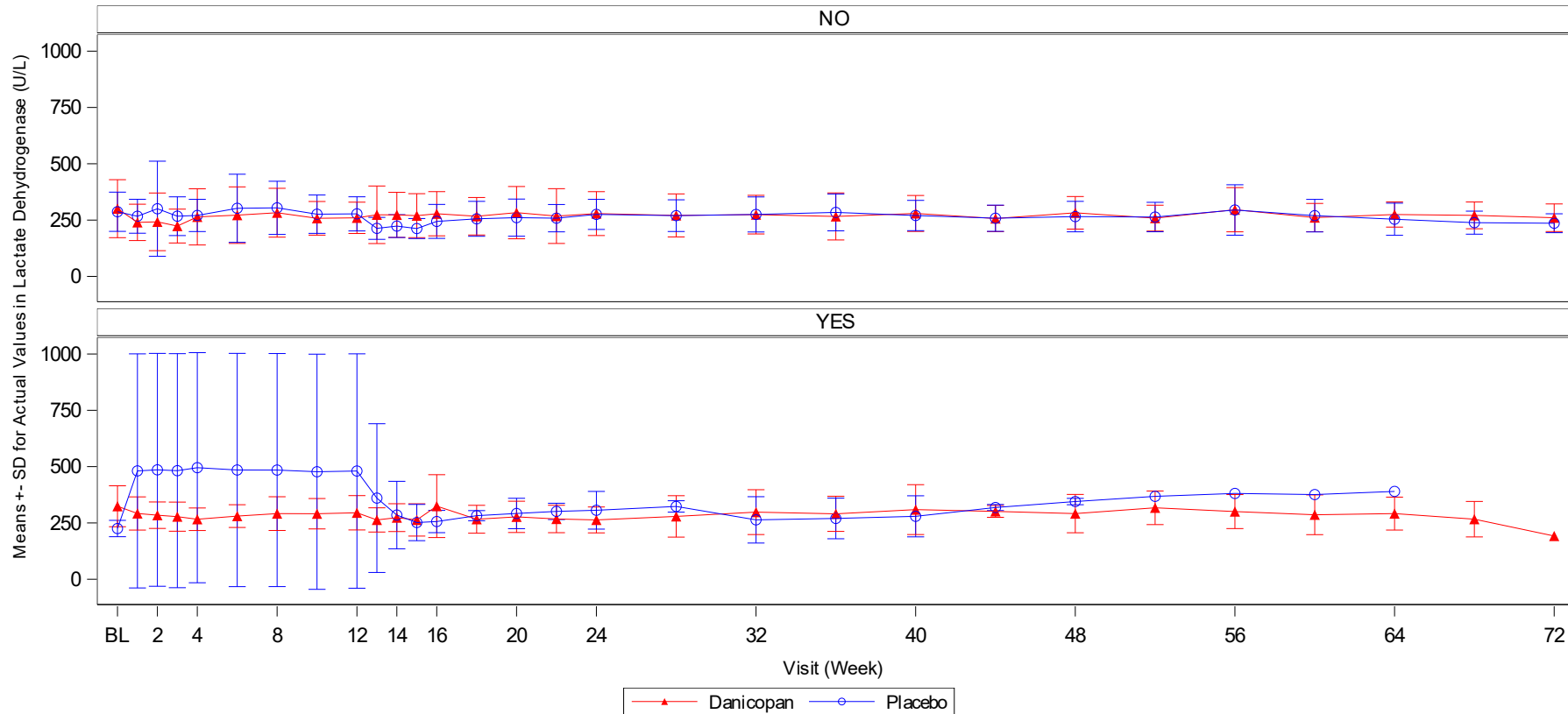
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Figure GERMANY.2.2.4.3.7

Plots of Mean \pm SD for Actual Values in Lactate Dehydrogenase (LDH) During 72 Weeks by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set



Baseline is defined as the average of all available assessments prior to the first dose of study drug.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium \geq 6 mmol/L and LDH \geq 2x ULN are defined as having TTH.

Source: ADaM.ADLB

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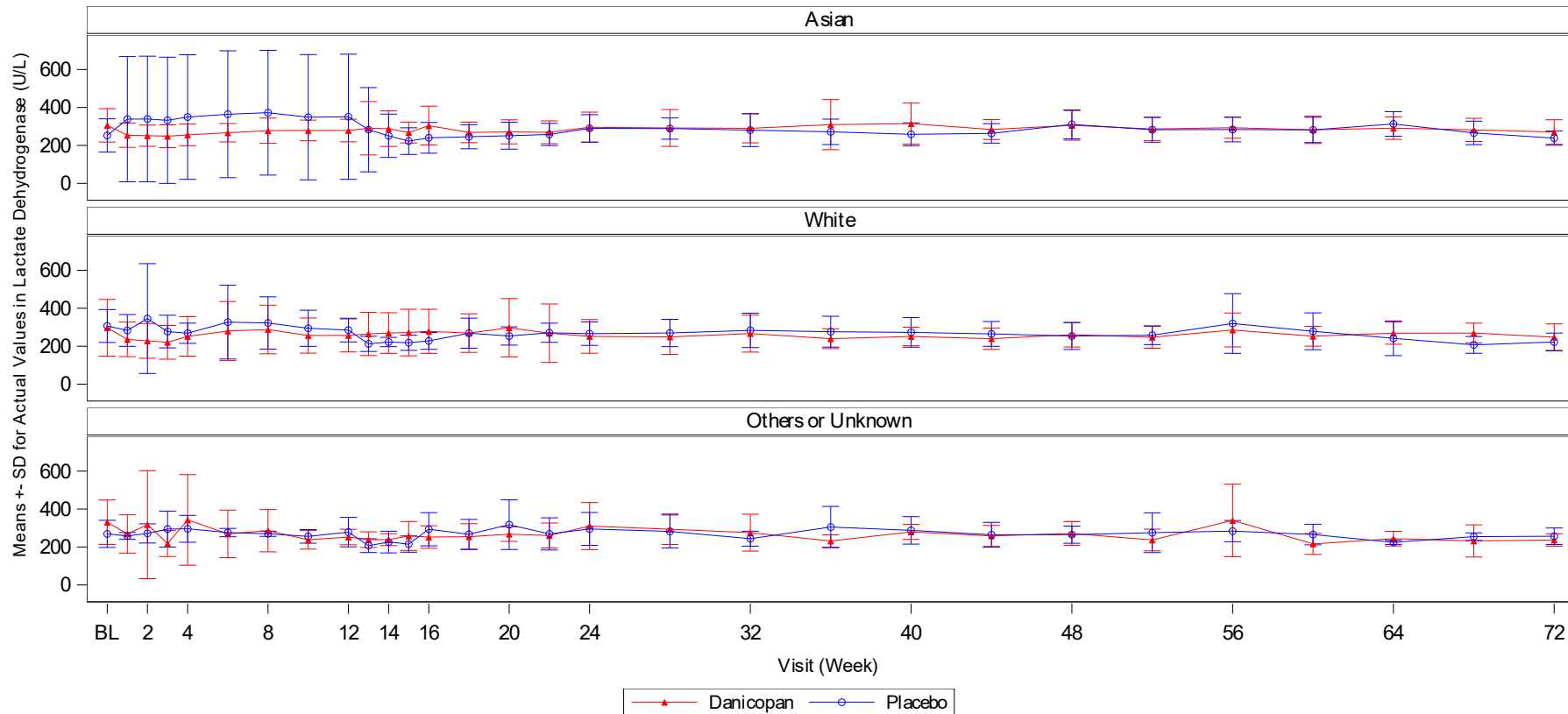
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Figure GERMANY.2.2.4.3.8
Plots of Mean \pm SD for Actual Values in Lactate Dehydrogenase (LDH) During 72 Weeks by Race
Modified Randomized Set



Baseline is defined as the average of all available assessments prior to the first dose of study drug.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.

Source: ADaM.ADLB

Run Date: 2024-04-26T15:31:13

Data cutoff date of 31Mar2023

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Table GERMANY.2.3.3.2
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Male

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-76.73 (11.011)	-45.10 (16.765)	-31.62 (20.125)	0.1268
	95% CI for LS MEAN	(-99.21, -54.24)	(-79.38, -10.82)	(-72.75, 9.51)	
Week 2	LS Mean (SEM)	-90.32 (37.333)	48.01 (58.311)	-138.33 (69.252)	0.0552
	95% CI for LS MEAN	(-166.66, -13.98)	(-71.24, 167.25)	(-279.95, 3.29)	
Week 3	LS Mean (SEM)	-99.68 (13.261)	-43.32 (20.569)	-56.36 (24.513)	0.0288
	95% CI for LS MEAN	(-126.78, -72.59)	(-85.37, -1.28)	(-106.46, -6.26)	
Week 4	LS Mean (SEM)	-58.92 (12.492)	-58.18 (21.820)	-0.74 (25.316)	0.9774
	95% CI for LS MEAN	(-88.06, -29.78)	(-108.52, -7.84)	(-59.22, 57.74)	
Week 6	LS Mean (SEM)	-43.68 (29.465)	1.68 (45.994)	-45.36 (54.641)	0.4132
	95% CI for LS MEAN	(-103.94, 16.57)	(-92.39, 95.74)	(-157.11, 66.39)	
Week 8	LS Mean (SEM)	-36.25 (20.393)	7.45 (31.120)	-43.70 (37.239)	0.2507
	95% CI for LS MEAN	(-78.01, 5.52)	(-56.39, 71.30)	(-120.05, 32.65)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Run Date: 2024-04-26T15:50:08

Data cutoff date of 31Mar2023

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Table GERMANY.2.3.3.2
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Male

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-47.97 (11.936)	-45.44 (18.285)	-2.53 (21.831)	0.9086
	95% CI for LS MEAN	(-72.52, -23.42)	(-83.11, -7.77)	(-47.49, 42.43)	
Week 12	LS Mean (SEM)	-53.87 (11.841)	-52.99 (18.329)	-0.88 (21.865)	0.9684
	95% CI for LS MEAN	(-78.12, -29.61)	(-90.55, -15.43)	(-45.68, 43.93)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Run Date: 2024-04-26T15:50:08

Data cutoff date of 31Mar2023

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Table GERMANY.2.3.3.2
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 16)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-37.84 (9.919)	-18.45 (13.930)	-19.39 (17.111)	0.2651
	95% CI for LS MEAN	(-57.96, -17.72)	(-46.78, 9.87)	(-54.15, 15.38)	
Week 2	LS Mean (SEM)	-43.68 (9.785)	-21.89 (14.227)	-21.80 (17.247)	0.2131
	95% CI for LS MEAN	(-63.41, -23.96)	(-50.57, 6.80)	(-56.57, 12.98)	
Week 3	LS Mean (SEM)	-49.37 (9.645)	-21.43 (14.154)	-27.93 (17.101)	0.1117
	95% CI for LS MEAN	(-68.97, -29.76)	(-50.20, 7.33)	(-62.69, 6.83)	
Week 4	LS Mean (SEM)	-32.98 (8.400)	2.74 (13.328)	-35.71 (15.634)	0.0287
	95% CI for LS MEAN	(-50.08, -15.87)	(-24.31, 29.79)	(-67.49, -3.94)	
Week 6	LS Mean (SEM)	-22.76 (10.763)	5.50 (15.599)	-28.26 (18.919)	0.1450
	95% CI for LS MEAN	(-44.66, -0.86)	(-26.27, 37.26)	(-66.78, 10.27)	
Week 8	LS Mean (SEM)	-6.20 (14.382)	-8.51 (21.378)	2.31 (25.768)	0.9291
	95% CI for LS MEAN	(-35.63, 23.23)	(-52.18, 35.16)	(-50.36, 54.99)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Run Date: 2024-04-26T15:50:08

Data cutoff date of 31Mar2023

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Table GERMANY.2.3.3.2
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 16)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-7.03 (10.794)	-5.04 (15.883)	-1.99 (19.176)	0.9180
	95% CI for LS MEAN	(-29.10, 15.03)	(-37.52, 27.43)	(-41.21, 37.22)	
Week 12	LS Mean (SEM)	-2.08 (9.883)	5.05 (13.982)	-7.13 (17.107)	0.6831
	95% CI for LS MEAN	(-23.11, 18.96)	(-24.96, 35.06)	(-43.76, 29.50)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Run Date: 2024-04-26T15:50:08

Data cutoff date of 31Mar2023

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Table GERMANY.2.3.3.2
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.4751
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Table GERMANY.2.3.3.3
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Age at Informed Consent
(< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

< 65 Years

Visit	Statistics	Danicopan (N = 40)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-58.46 (8.901)	-26.44 (11.842)	-32.02 (14.574)	0.0322
	95% CI for LS MEAN	(-76.26, -40.66)	(-50.18, -2.70)	(-61.23, -2.82)	
Week 2	LS Mean (SEM)	-72.16 (21.345)	11.61 (30.140)	-83.78 (36.847)	0.0267
	95% CI for LS MEAN	(-114.89, -29.44)	(-48.72, 71.95)	(-157.54, -10.01)	
Week 3	LS Mean (SEM)	-83.71 (9.976)	-26.54 (13.941)	-57.17 (16.951)	0.0014
	95% CI for LS MEAN	(-103.72, -63.70)	(-54.51, 1.44)	(-91.20, -23.15)	
Week 4	LS Mean (SEM)	-56.73 (8.596)	-22.74 (13.830)	-33.99 (16.125)	0.0528
	95% CI for LS MEAN	(-75.30, -38.16)	(-52.06, 6.58)	(-68.45, 0.48)	
Week 6	LS Mean (SEM)	-37.11 (17.359)	11.23 (24.452)	-48.34 (29.879)	0.1113
	95% CI for LS MEAN	(-71.87, -2.35)	(-37.75, 60.20)	(-108.19, 11.51)	
Week 8	LS Mean (SEM)	-27.38 (13.924)	13.63 (19.850)	-41.01 (24.107)	0.0949
	95% CI for LS MEAN	(-55.32, 0.56)	(-26.19, 53.46)	(-89.39, 7.38)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Run Date: 2024-04-26T15:50:09

Data cutoff date of 31Mar2023

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Table GERMANY.2.3.3.3
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Age at Informed Consent
(< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

< 65 Years

Visit	Statistics	Danicopan (N = 40)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-41.97 (9.267)	-11.45 (12.929)	-30.51 (15.701)	0.0571
	95% CI for LS MEAN	(-60.52, -23.41)	(-37.36, 14.46)	(-61.98, 0.95)	
Week 12	LS Mean (SEM)	-42.55 (8.994)	-12.36 (12.589)	-30.20 (15.262)	0.0542
	95% CI for LS MEAN	(-60.67, -24.44)	(-37.72, 13.01)	(-60.97, 0.57)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Run Date: 2024-04-26T15:50:09

Data cutoff date of 31Mar2023

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Table GERMANY.2.3.3.3
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Age at Informed Consent
(< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 5)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-51.76 (8.011)	-22.15 (14.351)	-29.61 (17.333)	0.1070
	95% CI for LS MEAN	(-68.84, -34.69)	(-52.73, 8.42)	(-66.36, 7.14)	
Week 2	LS Mean (SEM)	-40.97 (12.090)	-21.49 (21.497)	-19.48 (25.062)	0.4480
	95% CI for LS MEAN	(-66.57, -15.37)	(-67.03, 24.04)	(-72.48, 33.53)	
Week 3	LS Mean (SEM)	-38.47 (11.403)	-30.37 (21.239)	-8.10 (24.519)	0.7449
	95% CI for LS MEAN	(-62.52, -14.42)	(-74.90, 14.16)	(-59.49, 43.30)	
Week 4	LS Mean (SEM)	-15.78 (14.986)	-2.89 (26.702)	-12.89 (30.942)	0.6819
	95% CI for LS MEAN	(-47.32, 15.76)	(-59.10, 53.32)	(-77.97, 52.18)	
Week 6	LS Mean (SEM)	-15.22 (13.331)	-21.09 (23.729)	5.87 (27.579)	0.8340
	95% CI for LS MEAN	(-43.37, 12.93)	(-71.22, 29.04)	(-52.32, 64.07)	
Week 8	LS Mean (SEM)	5.47 (18.442)	-12.69 (32.905)	18.16 (37.983)	0.6385
	95% CI for LS MEAN	(-33.35, 44.29)	(-81.97, 56.58)	(-61.75, 98.07)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Run Date: 2024-04-26T15:50:09

Data cutoff date of 31Mar2023

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Table GERMANY.2.3.3.3
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Age at Informed Consent
(< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 5)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	11.69 (12.870)	-34.09 (22.879)	45.78 (26.522)	0.1049
	95% CI for LS MEAN	(-15.75, 39.12)	(-82.92, 14.74)	(-10.75, 102.30)	
Week 12	LS Mean (SEM)	14.40 (15.419)	-30.69 (26.574)	45.09 (30.832)	0.1677
	95% CI for LS MEAN	(-18.88, 47.68)	(-88.26, 26.87)	(-21.61, 111.79)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Run Date: 2024-04-26T15:50:09

Data cutoff date of 31Mar2023

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Table GERMANY.2.3.3.3

Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Age at Informed Consent
(< 65 and ≥ 65 Years)

Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.2585
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2x$ ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:50:09

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Table GERMANY.2.3.3.4
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Europe

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 13)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-53.26 (10.881)	-0.25 (14.266)	-53.01 (17.921)	0.0059
	95% CI for LS MEAN	(-75.42, -31.09)	(-29.36, 28.86)	(-89.56, -16.45)	
Week 2	LS Mean (SEM)	-57.23 (33.868)	63.60 (46.039)	-120.83 (57.132)	0.0417
	95% CI for LS MEAN	(-126.01, 11.55)	(-29.89, 157.10)	(-236.86, -4.80)	
Week 3	LS Mean (SEM)	-67.49 (13.814)	2.08 (18.733)	-69.57 (23.263)	0.0051
	95% CI for LS MEAN	(-95.56, -39.42)	(-35.99, 40.15)	(-116.84, -22.30)	
Week 4	LS Mean (SEM)	-39.73 (14.583)	17.04 (21.180)	-56.77 (25.696)	0.0714
	95% CI for LS MEAN	(-76.18, -3.28)	(-35.14, 69.21)	(-120.38, 6.85)	
Week 6	LS Mean (SEM)	-8.39 (26.354)	41.14 (35.306)	-49.53 (44.052)	0.2685
	95% CI for LS MEAN	(-61.87, 45.09)	(-30.58, 112.87)	(-138.99, 39.92)	
Week 8	LS Mean (SEM)	-20.39 (19.788)	40.31 (27.049)	-60.70 (33.473)	0.0786
	95% CI for LS MEAN	(-60.59, 19.82)	(-14.64, 95.26)	(-128.70, 7.31)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.4
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Europe

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 13)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-17.10 (14.834)	7.99 (19.823)	-25.09 (24.706)	0.3183
	95% CI for LS MEAN	(-47.42, 13.21)	(-32.58, 48.55)	(-75.63, 25.45)	
Week 12	LS Mean (SEM)	-15.79 (13.138)	10.14 (17.101)	-25.93 (21.508)	0.2378
	95% CI for LS MEAN	(-42.62, 11.04)	(-24.90, 45.18)	(-69.95, 18.09)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.4
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-62.65 (8.586)	-48.73 (13.226)	-13.92 (15.569)	0.3777
	95% CI for LS MEAN	(-80.02, -45.27)	(-75.61, -21.85)	(-45.55, 17.72)	
Week 2	LS Mean (SEM)	-69.51 (10.253)	-46.81 (16.362)	-22.70 (19.171)	0.2441
	95% CI for LS MEAN	(-90.27, -48.76)	(-79.99, -13.63)	(-61.58, 16.18)	
Week 3	LS Mean (SEM)	-74.82 (9.760)	-55.66 (15.670)	-19.15 (18.312)	0.3033
	95% CI for LS MEAN	(-94.65, -54.98)	(-87.55, -23.78)	(-56.43, 18.13)	
Week 4	LS Mean (SEM)	-50.83 (9.648)	-26.62 (15.942)	-24.21 (18.457)	0.1977
	95% CI for LS MEAN	(-70.35, -31.30)	(-58.89, 5.66)	(-61.60, 13.18)	
Week 6	LS Mean (SEM)	-50.10 (10.407)	-24.05 (17.083)	-26.05 (19.860)	0.1982
	95% CI for LS MEAN	(-71.21, -28.98)	(-58.70, 10.60)	(-66.36, 14.27)	
Week 8	LS Mean (SEM)	-20.18 (12.979)	-0.63 (21.326)	-19.55 (24.743)	0.4352
	95% CI for LS MEAN	(-46.56, 6.19)	(-44.07, 42.81)	(-69.96, 30.85)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.4
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-39.80 (8.730)	-35.05 (13.943)	-4.75 (16.254)	0.7724
	95% CI for LS MEAN	(-57.64, -21.96)	(-63.62, -6.49)	(-38.07, 28.58)	
Week 12	LS Mean (SEM)	-35.96 (8.983)	-34.18 (14.636)	-1.79 (16.986)	0.9168
	95% CI for LS MEAN	(-54.20, -17.73)	(-63.88, -4.47)	(-36.29, 32.72)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.4
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.1932
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Table GERMANY.2.3.3.5
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-49.06 (11.973)	-20.59 (16.976)	-28.47 (20.369)	0.1790
	95% CI for LS MEAN	(-73.94, -24.17)	(-56.26, 15.09)	(-71.24, 14.30)	
Week 2	LS Mean (SEM)	-61.50 (10.608)	-24.79 (16.006)	-36.71 (18.749)	0.0591
	95% CI for LS MEAN	(-83.06, -39.93)	(-57.37, 7.80)	(-74.91, 1.49)	
Week 3	LS Mean (SEM)	-69.36 (13.501)	-30.09 (20.059)	-39.27 (23.813)	0.1091
	95% CI for LS MEAN	(-96.79, -41.92)	(-70.96, 10.79)	(-87.80, 9.26)	
Week 4	LS Mean (SEM)	-44.91 (11.148)	-40.72 (18.217)	-4.20 (21.095)	0.8437
	95% CI for LS MEAN	(-67.80, -22.02)	(-78.04, -3.39)	(-47.47, 39.08)	
Week 6	LS Mean (SEM)	-10.12 (16.220)	-29.29 (24.522)	19.17 (29.129)	0.5161
	95% CI for LS MEAN	(-43.29, 23.05)	(-79.56, 20.98)	(-40.56, 78.90)	
Week 8	LS Mean (SEM)	-11.87 (17.047)	-15.31 (25.751)	3.44 (30.561)	0.9111
	95% CI for LS MEAN	(-46.57, 22.83)	(-67.80, 37.18)	(-58.88, 65.75)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.5
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Analysis Using a Mixed Model for Repeated Measures (MMRM) Modified Randomized Set

> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-16.31 (10.739)	-37.09 (15.667)	20.78 (18.512)	0.2733
	95% CI for LS MEAN	(-38.40, 5.79)	(-69.48, -4.69)	(-17.53, 59.09)	
Week 12	LS Mean (SEM)	-21.01 (12.876)	-17.39 (19.110)	-3.63 (22.671)	0.8745
	95% CI for LS MEAN	(-47.74, 5.72)	(-57.25, 22.48)	(-50.93, 43.67)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.5
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-58.04 (8.234)	-29.07 (12.298)	-28.98 (14.819)	0.0571
	95% CI for LS MEAN	(-74.67, -41.42)	(-53.87, -4.27)	(-58.87, 0.91)	
Week 2	LS Mean (SEM)	-64.08 (25.955)	27.31 (37.901)	-91.38 (45.942)	0.0528
	95% CI for LS MEAN	(-116.36, -11.79)	(-49.04, 103.65)	(-183.93, 1.17)	
Week 3	LS Mean (SEM)	-71.63 (9.659)	-26.80 (14.400)	-44.83 (17.362)	0.0137
	95% CI for LS MEAN	(-91.19, -52.07)	(-55.91, 2.31)	(-79.94, -9.72)	
Week 4	LS Mean (SEM)	-43.46 (10.452)	2.85 (16.592)	-46.32 (19.643)	0.0406
	95% CI for LS MEAN	(-67.19, -19.74)	(-34.02, 39.73)	(-90.22, -2.42)	
Week 6	LS Mean (SEM)	-44.54 (17.959)	28.79 (26.298)	-73.33 (31.858)	0.0261
	95% CI for LS MEAN	(-80.74, -8.35)	(-24.20, 81.78)	(-137.53, -9.13)	
Week 8	LS Mean (SEM)	-19.98 (14.792)	24.03 (22.000)	-44.01 (26.531)	0.1048
	95% CI for LS MEAN	(-49.89, 9.92)	(-20.39, 68.46)	(-97.61, 9.58)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.5
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-30.98 (10.476)	-0.58 (15.564)	-30.40 (18.792)	0.1131
	95% CI for LS MEAN	(-52.12, -9.84)	(-31.98, 30.81)	(-68.30, 7.51)	
Week 12	LS Mean (SEM)	-29.11 (9.568)	-11.79 (14.126)	-17.32 (17.096)	0.3197
	95% CI for LS MEAN	(-48.69, -9.52)	(-40.72, 17.14)	(-52.31, 17.68)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.5
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.3952
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.6
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-69.53 (8.382)	-19.83 (13.041)	-49.70 (15.643)	0.0027
	95% CI for LS MEAN	(-86.40, -52.65)	(-46.10, 6.45)	(-81.20, -18.20)	
Week 2	LS Mean (SEM)	-78.15 (24.503)	30.64 (38.769)	-108.78 (45.898)	0.0219
	95% CI for LS MEAN	(-127.44, -28.85)	(-47.36, 108.63)	(-201.12, -16.45)	
Week 3	LS Mean (SEM)	-81.57 (11.001)	-39.46 (17.464)	-42.11 (20.718)	0.0481
	95% CI for LS MEAN	(-103.74, -59.40)	(-74.64, -4.28)	(-83.84, -0.37)	
Week 4	LS Mean (SEM)	-60.97 (12.769)	2.98 (22.345)	-63.96 (25.808)	0.0498
	95% CI for LS MEAN	(-93.73, -28.21)	(-51.78, 57.75)	(-127.84, -0.07)	
Week 6	LS Mean (SEM)	-47.42 (19.093)	28.06 (29.917)	-75.48 (35.538)	0.0390
	95% CI for LS MEAN	(-85.83, -9.01)	(-32.15, 88.28)	(-147.00, -3.97)	
Week 8	LS Mean (SEM)	-28.76 (15.637)	40.07 (24.934)	-68.82 (29.491)	0.0238
	95% CI for LS MEAN	(-60.20, 2.69)	(-10.06, 90.20)	(-128.11, -9.54)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening), baseline LDH, study visit and study visit by treatment group interaction.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.6
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-39.40 (11.130)	-12.29 (17.015)	-27.10 (20.401)	0.1911
	95% CI for LS MEAN	(-61.83, -16.97)	(-46.64, 22.05)	(-68.26, 14.05)	
Week 12	LS Mean (SEM)	-53.23 (9.813)	-11.36 (15.218)	-41.86 (18.195)	0.0271
	95% CI for LS MEAN	(-73.09, -33.36)	(-42.21, 19.48)	(-78.72, -5.00)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening), baseline LDH, study visit and study visit by treatment group interaction.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Table GERMANY.2.3.3.6
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 11)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-40.87 (10.217)	-23.73 (13.279)	-17.15 (16.047)	0.2938
	95% CI for LS MEAN	(-61.65, -20.10)	(-50.84, 3.39)	(-49.93, 15.64)	
Week 2	LS Mean (SEM)	-43.79 (11.433)	-14.27 (15.512)	-29.52 (18.724)	0.1255
	95% CI for LS MEAN	(-67.10, -20.49)	(-45.92, 17.38)	(-67.78, 8.73)	
Week 3	LS Mean (SEM)	-58.00 (11.981)	-4.37 (16.495)	-53.62 (19.855)	0.0126
	95% CI for LS MEAN	(-82.68, -33.31)	(-38.40, 29.66)	(-94.66, -12.59)	
Week 4	LS Mean (SEM)	-22.13 (12.033)	-5.75 (17.045)	-16.38 (20.341)	0.4293
	95% CI for LS MEAN	(-47.05, 2.80)	(-41.04, 29.55)	(-58.59, 25.82)	
Week 6	LS Mean (SEM)	-12.57 (12.155)	-17.25 (16.701)	4.69 (20.110)	0.8182
	95% CI for LS MEAN	(-37.93, 12.80)	(-52.16, 17.65)	(-37.45, 46.83)	
Week 8	LS Mean (SEM)	-4.30 (14.174)	-25.24 (19.761)	20.94 (23.926)	0.3901
	95% CI for LS MEAN	(-33.50, 24.89)	(-65.93, 15.45)	(-28.42, 70.29)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening), baseline LDH, study visit and study visit by treatment group interaction.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.6
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 11)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-9.06 (10.283)	-16.36 (14.104)	7.30 (16.826)	0.6684
	95% CI for LS MEAN	(-30.29, 12.16)	(-45.49, 12.76)	(-27.52, 42.12)	
Week 12	LS Mean (SEM)	14.95 (13.652)	-16.59 (18.724)	31.54 (22.704)	0.1877
	95% CI for LS MEAN	(-14.30, 44.20)	(-56.85, 23.67)	(-17.42, 80.51)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening), baseline LDH, study visit and study visit by treatment group interaction.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.6
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by the Randomization
Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.4654
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening), baseline LDH, study visit and study visit by treatment group interaction.

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.7
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 20)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-38.54 (17.703)	-15.89 (22.021)	-22.66 (27.867)	0.4254
	95% CI for LS MEAN	(-75.29, -1.80)	(-61.69, 29.92)	(-80.64, 35.32)	
Week 2	LS Mean (SEM)	-50.94 (41.145)	46.04 (53.079)	-96.98 (66.982)	0.1581
	95% CI for LS MEAN	(-135.00, 33.11)	(-62.40, 154.48)	(-233.84, 39.88)	
Week 3	LS Mean (SEM)	-60.84 (17.438)	1.88 (23.222)	-62.72 (28.703)	0.0392
	95% CI for LS MEAN	(-96.90, -24.78)	(-46.07, 49.83)	(-122.06, -3.39)	
Week 4	LS Mean (SEM)	-39.24 (31.641)	51.58 (42.750)	-90.82 (53.017)	0.1289
	95% CI for LS MEAN	(-113.85, 35.36)	(-48.49, 151.65)	(-215.31, 33.67)	
Week 6	LS Mean (SEM)	-10.89 (30.050)	29.86 (39.004)	-40.75 (49.012)	0.4129
	95% CI for LS MEAN	(-72.52, 50.75)	(-50.07, 109.80)	(-141.24, 59.75)	
Week 8	LS Mean (SEM)	-1.31 (27.834)	24.51 (36.470)	-25.82 (45.629)	0.5764
	95% CI for LS MEAN	(-58.56, 55.94)	(-50.39, 99.41)	(-119.61, 67.98)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.7
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 20)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	2.57 (17.007)	7.58 (21.888)	-5.00 (27.327)	0.8567
	95% CI for LS MEAN	(-33.10, 38.24)	(-38.37, 53.52)	(-62.38, 52.37)	
Week 12	LS Mean (SEM)	-4.79 (19.153)	6.48 (24.503)	-11.28 (30.716)	0.7196
	95% CI for LS MEAN	(-46.12, 36.54)	(-46.52, 59.49)	(-77.76, 55.21)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.7
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 13)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-62.51 (6.653)	-33.98 (10.632)	-28.53 (12.433)	0.0265
	95% CI for LS MEAN	(-75.89, -49.13)	(-55.41, -12.56)	(-53.57, -3.48)	
Week 2	LS Mean (SEM)	-69.91 (7.758)	-27.75 (12.878)	-42.16 (14.957)	0.0071
	95% CI for LS MEAN	(-85.53, -54.30)	(-53.67, -1.84)	(-72.27, -12.06)	
Week 3	LS Mean (SEM)	-75.54 (7.386)	-43.21 (12.127)	-32.32 (14.116)	0.0265
	95% CI for LS MEAN	(-90.38, -60.69)	(-67.60, -18.82)	(-60.71, -3.93)	
Week 4	LS Mean (SEM)	-47.50 (8.341)	-29.68 (13.694)	-17.82 (15.972)	0.2702
	95% CI for LS MEAN	(-64.27, -30.72)	(-57.22, -2.13)	(-49.95, 14.31)	
Week 6	LS Mean (SEM)	-43.69 (10.607)	-10.44 (17.383)	-33.24 (20.307)	0.1085
	95% CI for LS MEAN	(-65.03, -22.35)	(-45.45, 24.56)	(-74.13, 7.64)	
Week 8	LS Mean (SEM)	-26.80 (9.309)	-13.29 (15.094)	-13.51 (17.657)	0.4483
	95% CI for LS MEAN	(-45.54, -8.06)	(-43.71, 17.13)	(-49.09, 22.07)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.7
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 13)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-40.46 (7.605)	-32.68 (12.335)	-7.78 (14.411)	0.5922
	95% CI for LS MEAN	(-55.79, -25.12)	(-57.58, -7.77)	(-36.87, 21.31)	
Week 12	LS Mean (SEM)	-31.31 (8.591)	-33.75 (14.037)	2.44 (16.385)	0.8821
	95% CI for LS MEAN	(-48.63, -13.98)	(-62.09, -5.42)	(-30.63, 35.52)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.7
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.6005
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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Table GERMANY.2.3.3.8
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 3)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-36.25 (32.490)	-26.82 (28.751)	-9.43 (51.876)	0.8607
	95% CI for LS MEAN	(-113.56, 41.06)	(-89.93, 36.30)	(-130.91, 112.05)	
Week 2	LS Mean (SEM)	-41.30 (32.083)	-20.48 (28.751)	-20.82 (51.565)	0.6981
	95% CI for LS MEAN	(-118.16, 35.55)	(-83.60, 42.63)	(-141.96, 100.32)	
Week 3	LS Mean (SEM)	-47.80 (32.083)	-25.48 (28.751)	-22.32 (51.565)	0.6777
	95% CI for LS MEAN	(-124.66, 29.05)	(-88.60, 37.63)	(-143.46, 98.82)	
Week 4	LS Mean (SEM)	-59.30 (32.083)	-7.82 (28.751)	-51.49 (51.565)	0.3503
	95% CI for LS MEAN	(-136.16, 17.55)	(-70.93, 55.30)	(-172.63, 69.65)	
Week 6	LS Mean (SEM)	-45.30 (32.083)	-21.48 (28.751)	-23.82 (51.565)	0.6577
	95% CI for LS MEAN	(-122.16, 31.55)	(-84.60, 41.63)	(-144.96, 97.32)	
Week 8	LS Mean (SEM)	-35.93 (32.289)	-21.82 (28.751)	-14.12 (51.601)	0.7921
	95% CI for LS MEAN	(-113.20, 41.34)	(-84.93, 41.30)	(-135.48, 107.25)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.8
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 3)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-34.80 (32.083)	-31.82 (28.751)	-2.99 (51.565)	0.9554
	95% CI for LS MEAN	(-111.66, 42.05)	(-94.93, 31.30)	(-124.13, 118.15)	
Week 12	LS Mean (SEM)	-30.55 (32.083)	-27.48 (28.751)	-3.07 (51.565)	0.9541
	95% CI for LS MEAN	(-107.41, 46.30)	(-90.60, 35.63)	(-124.21, 118.07)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:50:14

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Table GERMANY.2.3.3.8
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 48)	Placebo (N = 22)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-59.84 (7.626)	-25.24 (10.635)	-34.60 (12.897)	0.0093
	95% CI for LS MEAN	(-75.05, -44.63)	(-46.49, -3.99)	(-60.36, -8.83)	
Week 2	LS Mean (SEM)	-68.58 (18.159)	10.63 (26.731)	-79.21 (32.242)	0.0166
	95% CI for LS MEAN	(-104.81, -32.35)	(-42.71, 63.97)	(-143.55, -14.87)	
Week 3	LS Mean (SEM)	-76.19 (8.847)	-25.61 (12.989)	-50.58 (15.577)	0.0019
	95% CI for LS MEAN	(-93.87, -58.52)	(-51.56, 0.34)	(-81.71, -19.45)	
Week 4	LS Mean (SEM)	-43.61 (9.517)	-6.37 (15.036)	-37.24 (17.680)	0.0522
	95% CI for LS MEAN	(-64.04, -23.17)	(-38.22, 25.49)	(-74.89, 0.41)	
Week 6	LS Mean (SEM)	-30.50 (15.244)	8.47 (22.389)	-38.97 (26.998)	0.1537
	95% CI for LS MEAN	(-60.95, -0.06)	(-36.25, 53.19)	(-92.91, 14.96)	
Week 8	LS Mean (SEM)	-17.23 (12.833)	13.92 (19.144)	-31.15 (22.948)	0.1796
	95% CI for LS MEAN	(-42.88, 8.42)	(-24.33, 52.17)	(-77.02, 14.72)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Run Date: 2024-04-26T15:50:14

Data cutoff date of 31Mar2023

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Table GERMANY.2.3.3.8
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 48)	Placebo (N = 22)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-27.62 (8.649)	-12.65 (12.504)	-14.97 (15.053)	0.3240
	95% CI for LS MEAN	(-44.89, -10.34)	(-37.66, 12.36)	(-45.07, 15.14)	
Week 12	LS Mean (SEM)	-25.63 (8.939)	-13.31 (12.905)	-12.32 (15.553)	0.4324
	95% CI for LS MEAN	(-43.62, -7.65)	(-39.32, 12.70)	(-43.67, 19.02)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.8
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.3914
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:50:14

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Table GERMANY.2.3.3.9
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-51.87 (7.376)	-32.77 (10.657)	-19.11 (12.193)	0.1290
	95% CI for LS MEAN	(-66.99, -36.76)	(-54.64, -10.89)	(-44.15, 5.94)	
Week 2	LS Mean (SEM)	-53.96 (9.539)	-32.10 (14.402)	-21.86 (16.698)	0.2025
	95% CI for LS MEAN	(-73.52, -34.39)	(-61.71, -2.49)	(-56.25, 12.53)	
Week 3	LS Mean (SEM)	-56.82 (9.471)	-39.54 (14.293)	-17.28 (16.565)	0.3060
	95% CI for LS MEAN	(-76.16, -37.48)	(-68.79, -10.30)	(-51.23, 16.68)	
Week 4	LS Mean (SEM)	-50.68 (10.758)	-20.54 (16.109)	-30.14 (18.870)	0.1229
	95% CI for LS MEAN	(-72.76, -28.60)	(-53.69, 12.61)	(-69.02, 8.74)	
Week 6	LS Mean (SEM)	-38.46 (12.527)	-4.10 (19.199)	-34.36 (22.494)	0.1374
	95% CI for LS MEAN	(-64.00, -12.91)	(-43.31, 35.11)	(-80.34, 11.63)	
Week 8	LS Mean (SEM)	-26.68 (10.488)	4.90 (15.646)	-31.58 (18.279)	0.0955
	95% CI for LS MEAN	(-48.11, -5.25)	(-27.15, 36.96)	(-69.09, 5.93)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.9
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-27.64 (10.457)	-21.43 (15.735)	-6.21 (18.390)	0.7384
	95% CI for LS MEAN	(-49.03, -6.25)	(-53.70, 10.83)	(-43.96, 31.55)	
Week 12	LS Mean (SEM)	-26.82 (10.587)	-18.77 (16.092)	-8.05 (18.748)	0.6707
	95% CI for LS MEAN	(-48.41, -5.23)	(-51.64, 14.11)	(-46.41, 30.30)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.9
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 11)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-52.27 (13.420)	-14.33 (20.494)	-37.94 (24.410)	0.1312
	95% CI for LS MEAN	(-79.69, -24.85)	(-56.30, 27.64)	(-87.91, 12.03)	
Week 2	LS Mean (SEM)	-64.61 (32.015)	48.40 (51.075)	-113.01 (60.258)	0.0688
	95% CI for LS MEAN	(-129.52, 0.30)	(-55.15, 151.95)	(-235.19, 9.16)	
Week 3	LS Mean (SEM)	-73.68 (12.312)	-17.38 (19.665)	-56.30 (23.152)	0.0212
	95% CI for LS MEAN	(-98.83, -48.52)	(-57.54, 22.79)	(-103.59, -9.01)	
Week 4	LS Mean (SEM)	-41.08 (12.891)	-11.49 (24.819)	-29.59 (27.955)	0.3247
	95% CI for LS MEAN	(-72.75, -9.40)	(-69.32, 46.34)	(-95.56, 36.39)	
Week 6	LS Mean (SEM)	-11.72 (24.950)	25.82 (39.505)	-37.54 (46.702)	0.4269
	95% CI for LS MEAN	(-62.36, 38.92)	(-54.36, 106.00)	(-132.33, 57.25)	
Week 8	LS Mean (SEM)	-8.92 (23.120)	5.51 (37.284)	-14.43 (43.849)	0.7442
	95% CI for LS MEAN	(-56.05, 38.22)	(-70.42, 81.44)	(-103.76, 74.90)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.9
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 11)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-14.64 (15.394)	-4.95 (24.589)	-9.69 (28.969)	0.7411
	95% CI for LS MEAN	(-46.50, 17.23)	(-55.89, 45.99)	(-69.69, 50.31)	
Week 12	LS Mean (SEM)	-13.84 (14.952)	-18.78 (23.981)	4.95 (28.221)	0.8625
	95% CI for LS MEAN	(-44.81, 17.13)	(-68.46, 30.89)	(-53.51, 63.40)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Run Date: 2024-04-26T15:50:16

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Table GERMANY.2.3.3.9
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Others/Unknown

Visit	Statistics	Danicopan (N = 6)	Placebo (N = 5)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-79.85 (25.495)	-43.63 (26.907)	-36.23 (37.005)	0.3316
	95% CI for LS MEAN	(-130.85, -28.85)	(-97.50, 10.25)	(-110.27, 37.82)	
Week 2	LS Mean (SEM)	-103.31 (21.616)	-17.73 (23.135)	-85.58 (31.598)	0.0091
	95% CI for LS MEAN	(-146.65, -59.97)	(-64.13, 28.67)	(-148.95, -22.22)	
Week 3	LS Mean (SEM)	-117.65 (21.616)	-1.54 (26.099)	-116.10 (33.840)	0.0012
	95% CI for LS MEAN	(-160.99, -74.30)	(-53.86, 50.77)	(-183.94, -48.27)	
Week 4	LS Mean (SEM)	-56.48 (21.616)	6.67 (23.135)	-63.15 (31.598)	0.0508
	95% CI for LS MEAN	(-99.82, -13.14)	(-39.73, 53.07)	(-126.52, 0.22)	
Week 6	LS Mean (SEM)	-88.98 (21.616)	-13.33 (23.135)	-75.65 (31.598)	0.0202
	95% CI for LS MEAN	(-132.32, -45.64)	(-59.73, 33.07)	(-139.02, -12.28)	
Week 8	LS Mean (SEM)	-49.48 (21.616)	-16.92 (27.670)	-32.56 (34.579)	0.3503
	95% CI for LS MEAN	(-92.82, -6.14)	(-72.28, 38.45)	(-101.81, 36.69)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.9
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Others/Unknown

Visit	Statistics	Danicopan (N = 6)	Placebo (N = 5)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-86.31 (21.616)	-33.53 (23.135)	-52.78 (31.598)	0.1007
	95% CI for LS MEAN	(-129.65, -42.97)	(-79.93, 12.87)	(-116.15, 10.58)	
Week 12	LS Mean (SEM)	-64.15 (21.616)	-10.93 (23.135)	-53.22 (31.598)	0.0980
	95% CI for LS MEAN	(-107.49, -20.80)	(-57.33, 35.47)	(-116.58, 10.15)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

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Table GERMANY.2.3.3.9
Change from Baseline in Lactate Dehydrogenase (LDH) (U/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.2996
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

Estimates are based on MMRM that includes treatment group, the observed randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL), baseline LDH, study visit and study visit by treatment group interaction. LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

P-value are from testing treatment difference of MMRM. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Source: ADaM.ADSL, ADaM.ADEFF

Data cutoff date of 31Mar2023

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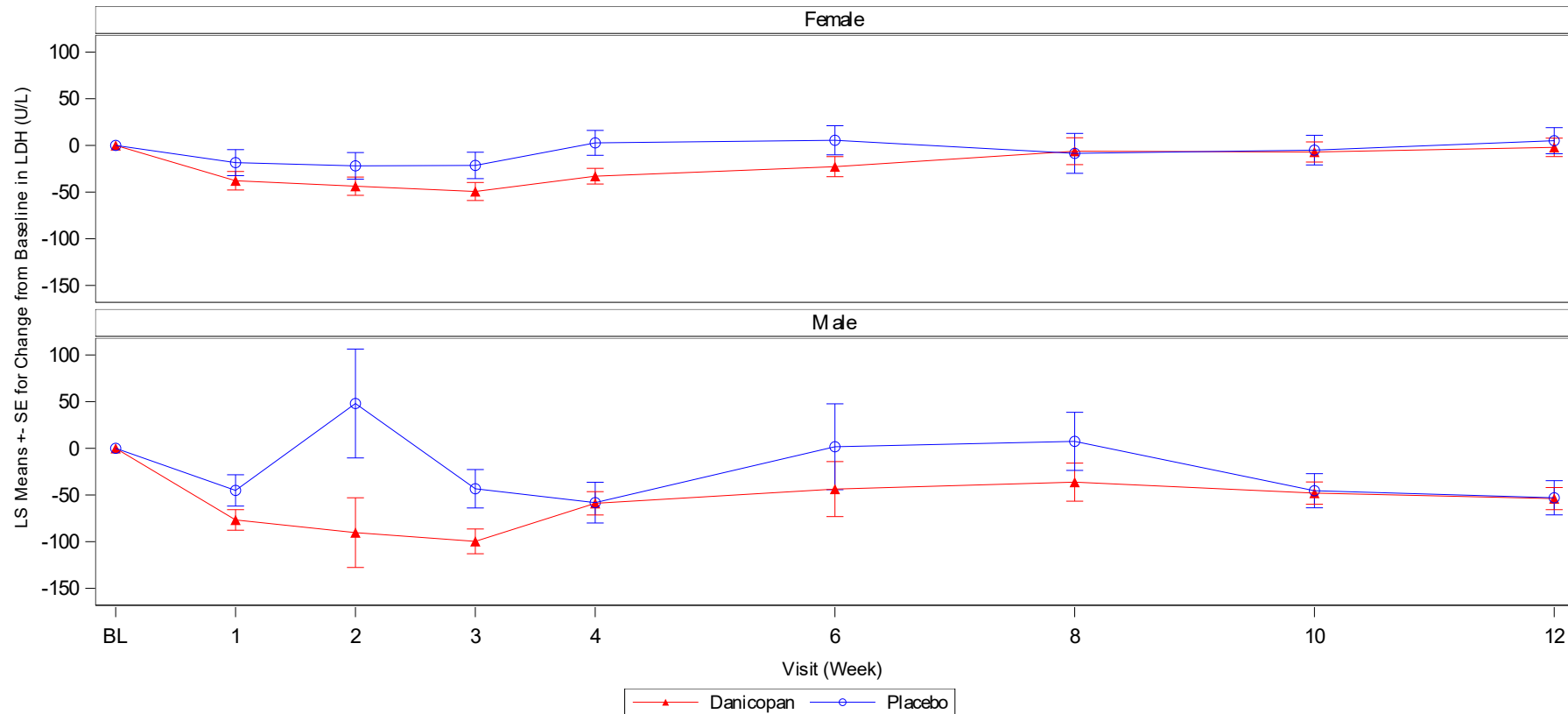
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Figure GERMANY.2.2.4.1.1

Plots of LS Means \pm SE for Change from Baseline in Lactate Dehydrogenase (LDH) During 12-Week of Treatment Period 1 by Sex Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium \geq 6 mmol/L and LDH \geq 2x ULN are defined as having TTH.

Source: ADaM.ADSL, ADaM.ADEFF

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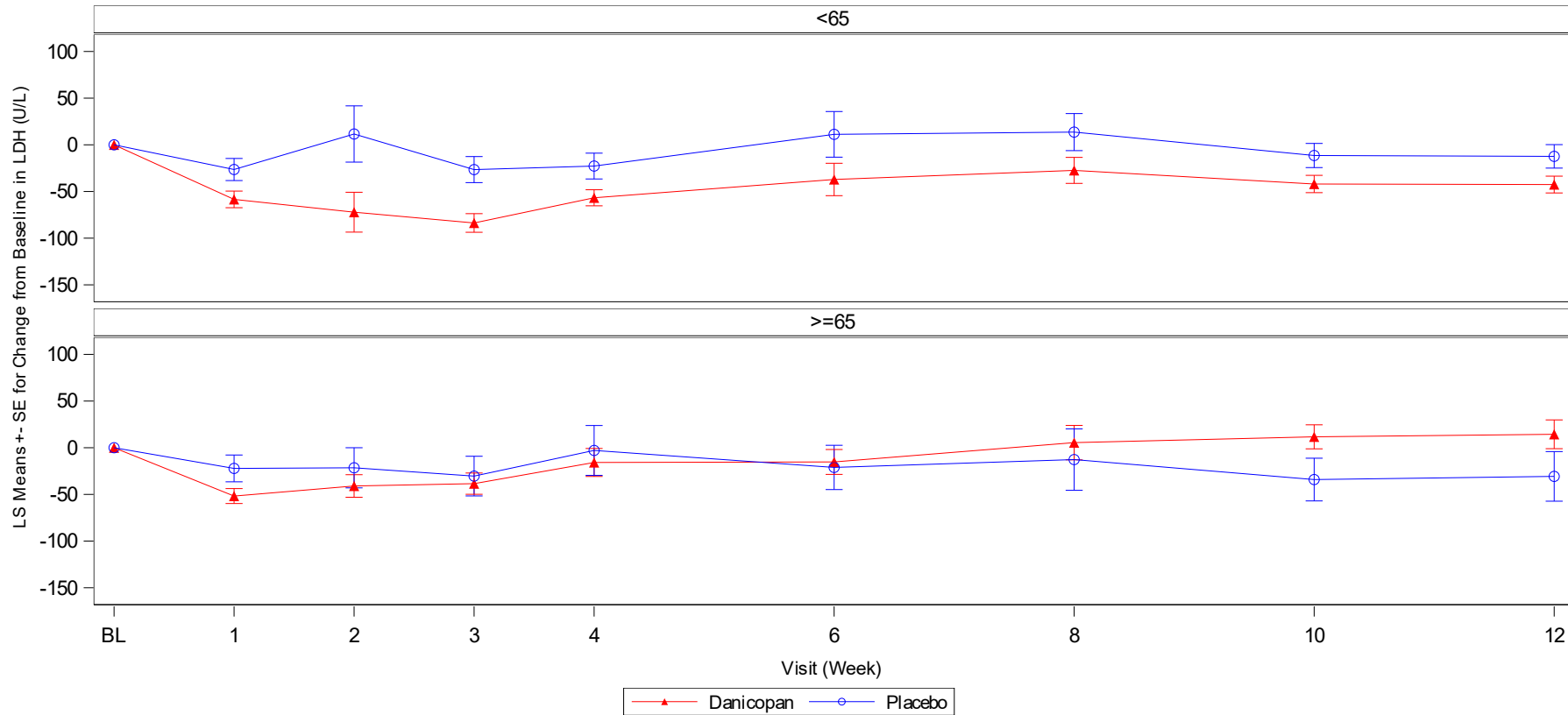
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Figure GERMANY.2.2.4.1.2
Plots of LS Means \pm SE for Change from Baseline in Lactate Dehydrogenase (LDH) During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and \geq 65 Years)
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium \geq 6 mmol/L and LDH \geq 2x ULN are defined as having TTH.

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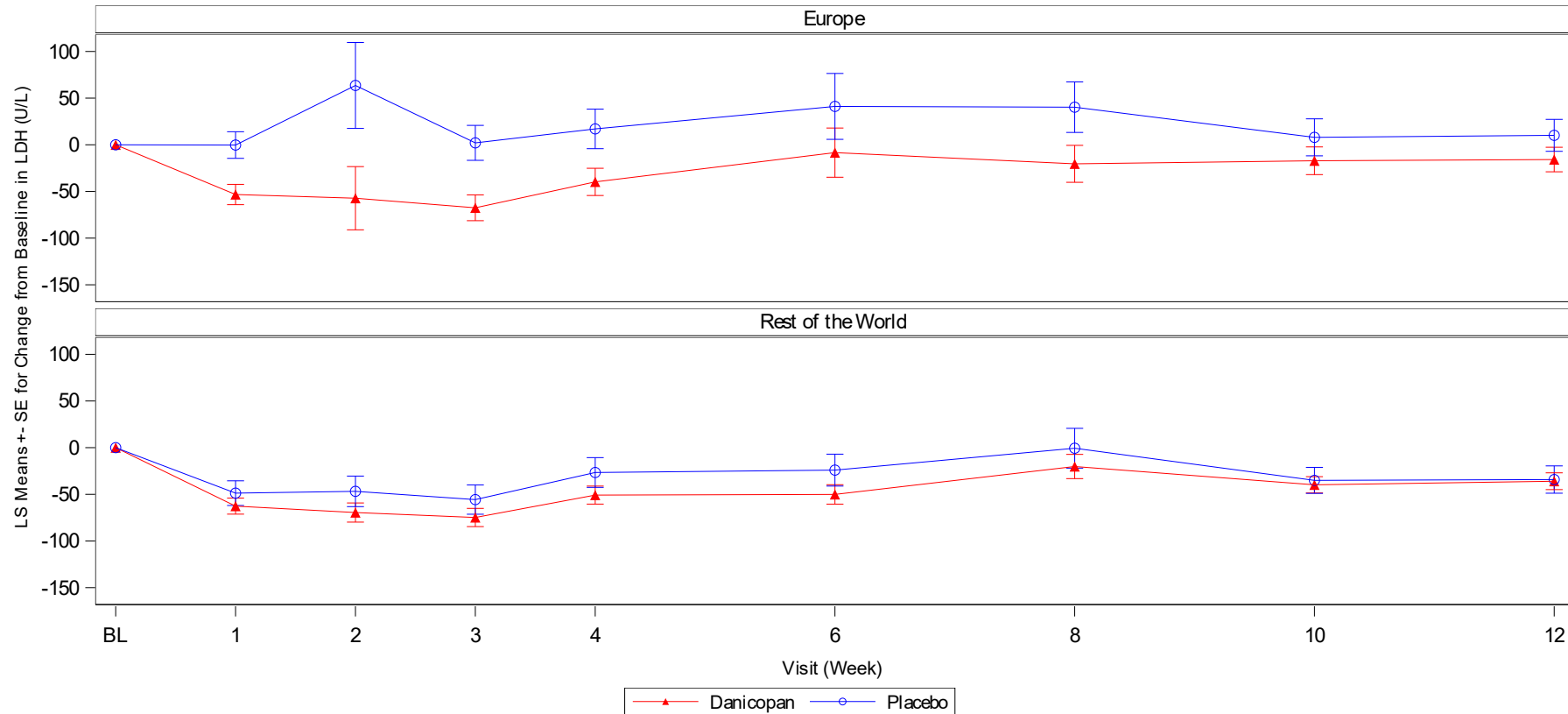
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Figure GERMANY.2.2.4.1.3

Plots of LS Means \pm SE for Change from Baseline in Lactate Dehydrogenase (LDH) During 12-Week of Treatment Period 1 by Region
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.

Source: ADaM.ADSL, ADaM.ADEFF

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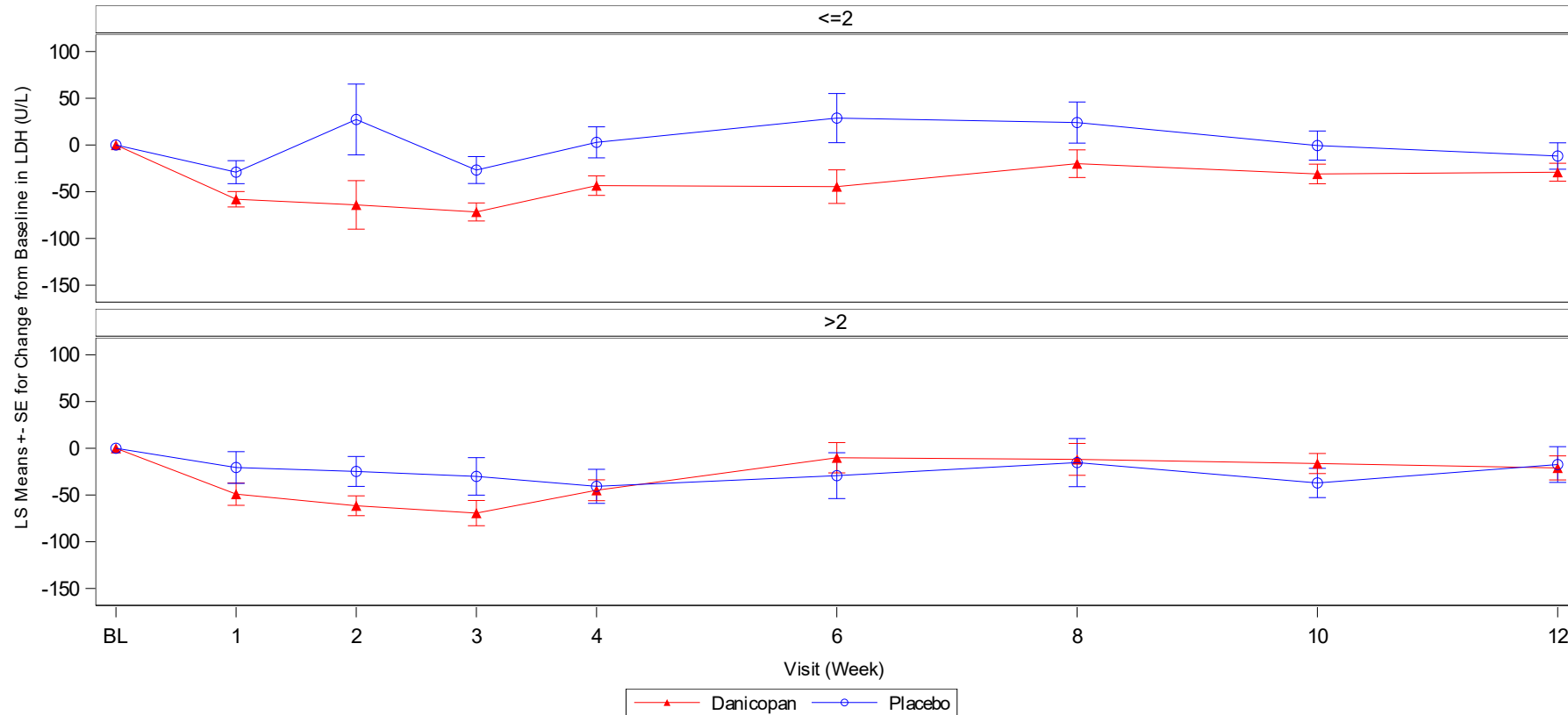
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Figure GERMANY.2.2.4.1.4

Plots of LS Means \pm SE for Change from Baseline in Lactate Dehydrogenase (LDH) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.

Source: ADaM.ADSL, ADaM.ADEFF

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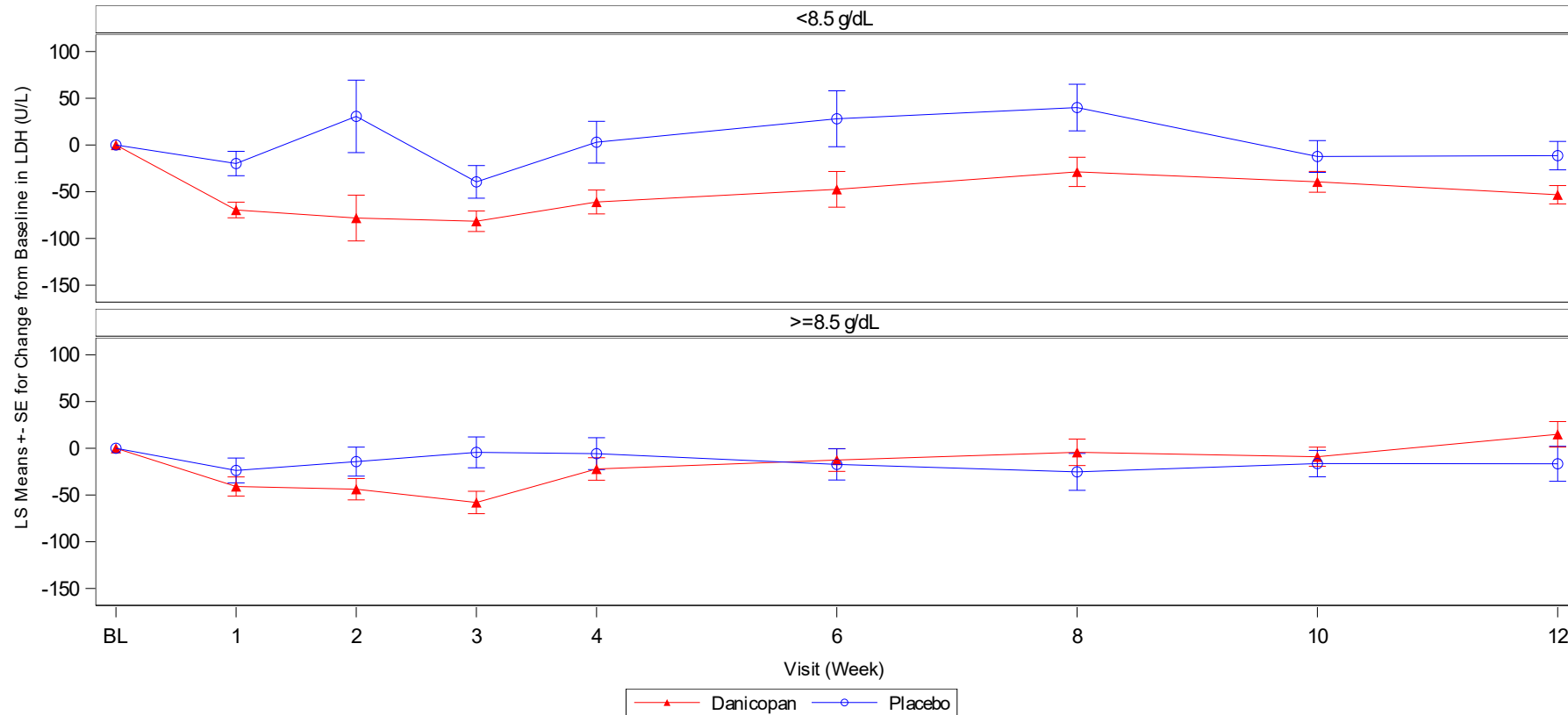
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Figure GERMANY.2.2.4.1.5

Plots of LS Means +- SE for Change from Baseline in Lactate Dehydrogenase (LDH) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH ≥ 2x ULN are defined as having TTH.

Source: ADaM.ADSL, ADaM.ADEFF

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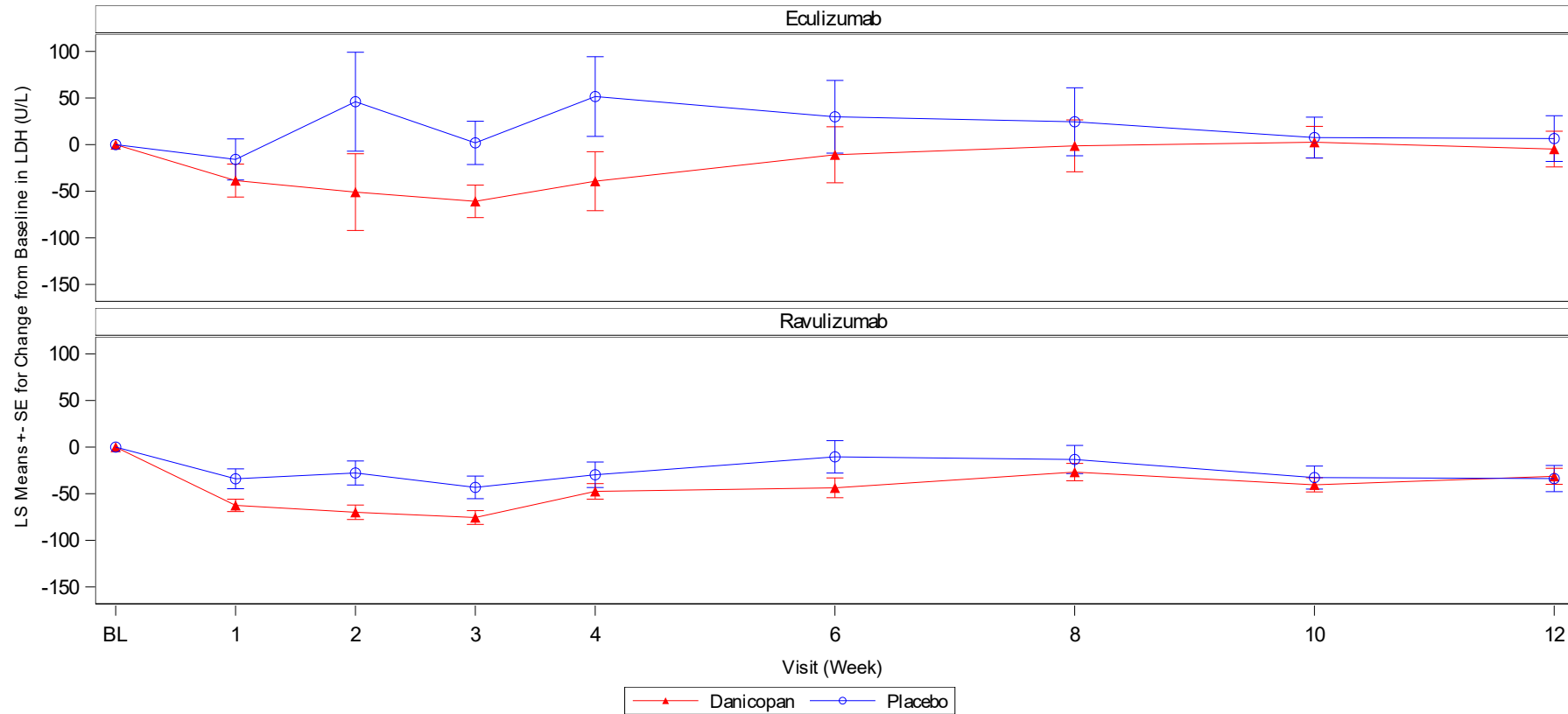
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Figure GERMANY.2.2.4.1.6

Plots of LS Means \pm SE for Change from Baseline in Lactate Dehydrogenase (LDH) During 12-Week of Treatment Period 1 by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium \geq 6 mmol/L and LDH \geq 2x ULN are defined as having TTH.

Source: ADaM.ADSL, ADaM.ADEFF

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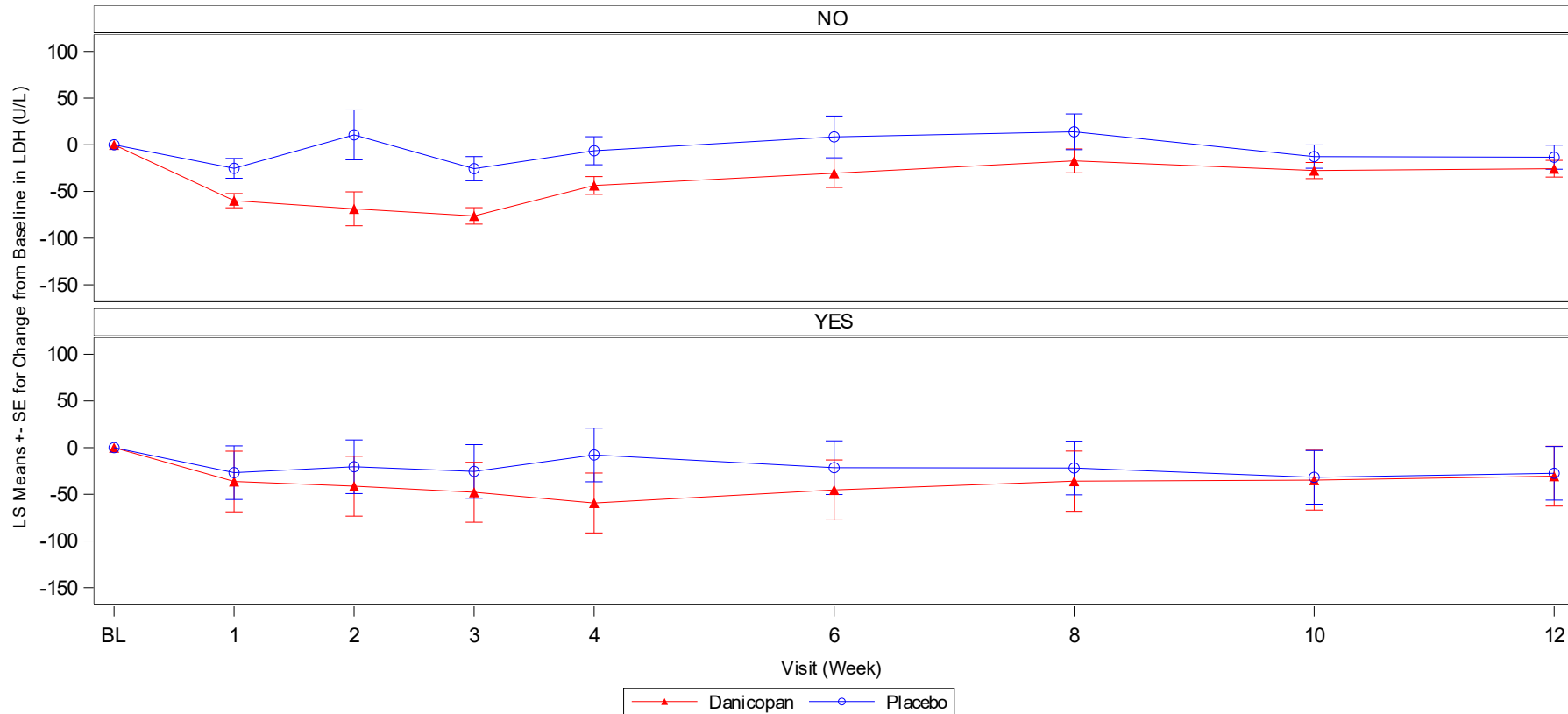
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Figure GERMANY.2.2.4.1.7

Plots of LS Means \pm SE for Change from Baseline in Lactate Dehydrogenase (LDH) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium ≥ 6 mmol/L and LDH $\geq 2 \times$ ULN are defined as having TTH.

Source: ADaM.ADSL, ADaM.ADEFF

Run Date: 2024-04-26T15:31:22

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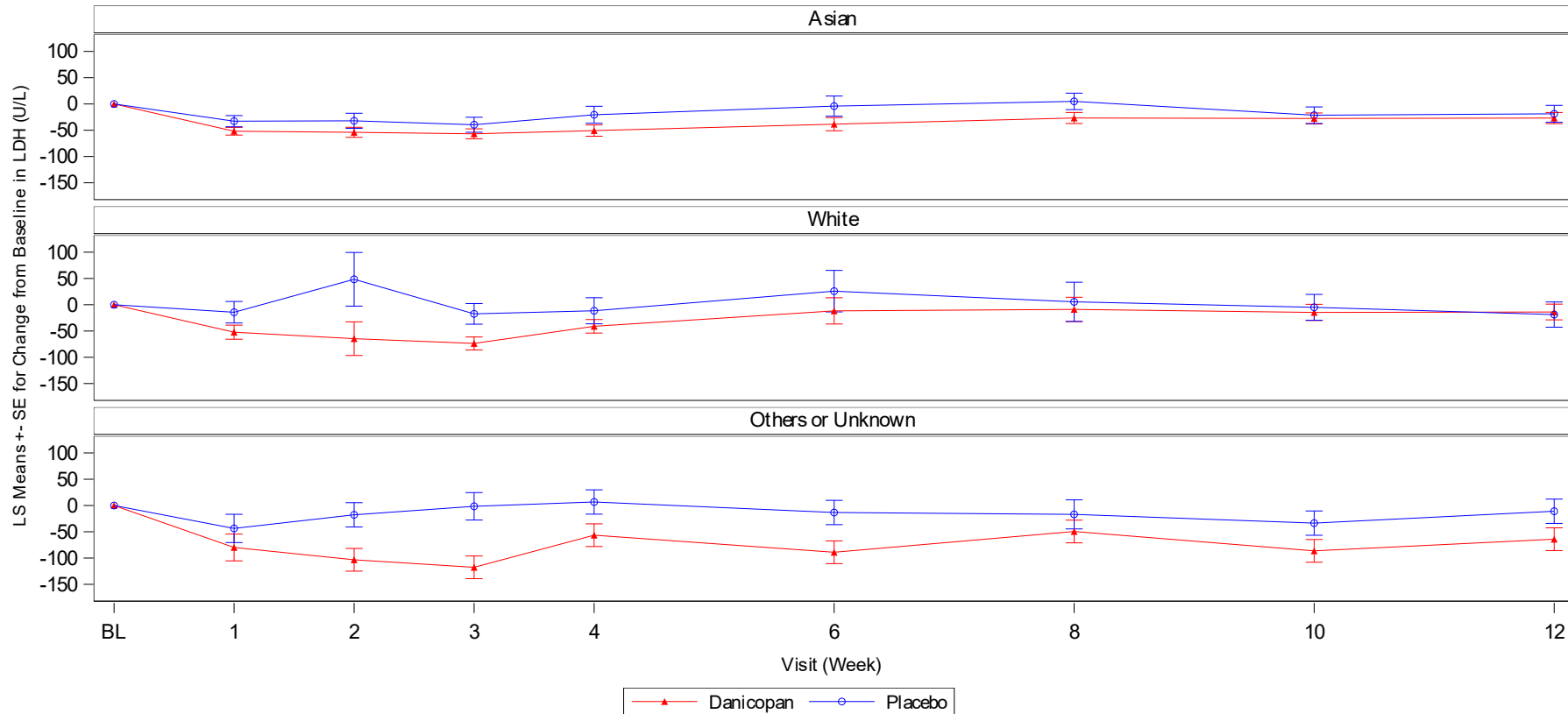
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Figure GERMANY.2.2.4.1.8
Plots of LS Means \pm SE for Change from Baseline in Lactate Dehydrogenase (LDH) During 12-Week of Treatment Period 1
by Race
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the average of all available assessments prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

LDH values in samples affected by table-top hemolysis (TTH) are not used in the analysis. Lab samples with serum potassium \geq 6 mmol/L and LDH \geq 2x ULN are defined as having TTH.

Source: ADaM.ADSL, ADaM.ADEFF

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Data cutoff date of 31Mar2023

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom

31.03.2023 1 Morbidität

1.4 Veränderung der absoluten Retikulozytenzahl gegenüber Baseline

Post-hoc Analysen Studie ALPHA

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Table GERMANY.2.6.4.1
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	57		25		82	
Mean	0.24762		0.21882		0.23884	
(SD)	(0.097187)		(0.118386)		(0.104215)	
Median	0.22100		0.18170		0.21080	
Min, Max	0.1094, 0.5295		0.0394, 0.5419		0.0394, 0.5419	
Week 1						
n	47	47	21	20		
Mean	0.16762	-0.07778	0.20783	0.00467		
(SD)	(0.060211)	(0.063655)	(0.094441)	(0.041052)		
Median	0.15920	-0.07870	0.21680	0.00515		
Min, Max	0.0553, 0.3200	-0.2457, 0.0183	0.0410, 0.4444	-0.0975, 0.0929		
Week 2						
n	53	53	22	21		
Mean	0.12379	-0.12713	0.22090	0.00328		
(SD)	(0.055940)	(0.099427)	(0.099564)	(0.032254)		
Median	0.11660	-0.10000	0.20655	-0.00350		
Min, Max	0.0388, 0.3474	-0.4371, 0.0089	0.0402, 0.4748	-0.0671, 0.0686		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.6.4.1
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	46	46	20	20		
Mean	0.11542	-0.12372	0.21005	-0.00401		
(SD)	(0.056660)	(0.092894)	(0.102806)	(0.037230)		
Median	0.10370	-0.10695	0.20555	-0.00325		
Min, Max	0.0400, 0.2506	-0.4571, 0.0148	0.0309, 0.5193	-0.1185, 0.0529		
Week 4						
n	50	50	21	21		
Mean	0.12274	-0.11785	0.20797	-0.00939		
(SD)	(0.055282)	(0.094813)	(0.115451)	(0.046095)		
Median	0.11095	-0.11495	0.18430	0.00000		
Min, Max	0.0326, 0.2846	-0.3445, 0.1083	0.0388, 0.5743	-0.1138, 0.0579		
Week 6						
n	47	47	22	22		
Mean	0.12972	-0.10393	0.22243	0.00019		
(SD)	(0.051650)	(0.075517)	(0.117152)	(0.033456)		
Median	0.12020	-0.08900	0.20610	0.00465		
Min, Max	0.0554, 0.3100	-0.3016, 0.0255	0.0343, 0.5254	-0.0716, 0.0611		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB
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Data cutoff date of 31Mar2023
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Table GERMANY.2.6.4.1
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	44	44	22	22		
Mean	0.13743	-0.09430	0.20960	-0.00099		
(SD)	(0.049966)	(0.075023)	(0.091024)	(0.044019)		
Median	0.12395	-0.08705	0.20910	-0.00065		
Min, Max	0.0682, 0.3244	-0.2995, 0.0651	0.0424, 0.4560	-0.0859, 0.0827		
Week 10						
n	37	37	20	20		
Mean	0.15441	-0.09617	0.22223	0.00855		
(SD)	(0.055124)	(0.076736)	(0.101002)	(0.047768)		
Median	0.13930	-0.07890	0.19670	0.01190		
Min, Max	0.0534, 0.3238	-0.2859, 0.0645	0.1180, 0.5817	-0.1417, 0.0855		
Week 12						
n	45	45	22	22		
Mean	0.15357	-0.09207	0.22759	0.00255		
(SD)	(0.057588)	(0.094676)	(0.113987)	(0.049782)		
Median	0.14120	-0.08000	0.21870	-0.00160		
Min, Max	0.0612, 0.3537	-0.3279, 0.0944	0.0383, 0.5375	-0.1279, 0.0948		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB
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Data cutoff date of 31Mar2023
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Table GERMANY.2.6.4.1
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	38	38	20	19		
Mean	0.15150	-0.08948	0.16338	-0.03884		
(SD)	(0.053531)	(0.092438)	(0.059702)	(0.075816)		
Median	0.15030	-0.06970	0.16360	-0.03110		
Min, Max	0.0495, 0.3100	-0.3539, 0.0460	0.0291, 0.3126	-0.2200, 0.0841		
Week 14						
n	40	40	22	22		
Mean	0.14868	-0.09760	0.12294	-0.08212		
(SD)	(0.046016)	(0.088251)	(0.041482)	(0.099235)		
Median	0.14770	-0.07780	0.12500	-0.07395		
Min, Max	0.0642, 0.2720	-0.3537, 0.0100	0.0241, 0.2053	-0.3603, 0.0501		
Week 15						
n	39	39	19	18		
Mean	0.14814	-0.08322	0.11805	-0.08716		
(SD)	(0.046821)	(0.089197)	(0.042005)	(0.078150)		
Median	0.14320	-0.07680	0.11600	-0.08595		
Min, Max	0.0736, 0.2517	-0.3686, 0.0437	0.0328, 0.2208	-0.2673, 0.0599		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:53:38

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Post-hoc Analysen Studie ALPHA

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Table GERMANY.2.6.4.1
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	42	42	23	22		
Mean	0.15445	-0.08278	0.12050	-0.08919		
(SD)	(0.047347)	(0.084939)	(0.056118)	(0.077403)		
Median	0.15270	-0.05745	0.10640	-0.08635		
Min, Max	0.0819, 0.2986	-0.3234, 0.0749	0.0315, 0.3029	-0.2719, 0.0639		
Week 18						
n	39	39	18	17		
Mean	0.16909	-0.06666	0.13101	-0.07636		
(SD)	(0.060858)	(0.074878)	(0.056352)	(0.075265)		
Median	0.16380	-0.05230	0.11945	-0.06500		
Min, Max	0.0761, 0.3164	-0.2201, 0.0800	0.0461, 0.2377	-0.2256, 0.0396		
Week 20						
n	41	41	17	16		
Mean	0.15440	-0.09112	0.13674	-0.08214		
(SD)	(0.056947)	(0.079470)	(0.074322)	(0.061538)		
Median	0.14590	-0.07340	0.11940	-0.07755		
Min, Max	0.0538, 0.2949	-0.3087, 0.0712	0.0362, 0.3278	-0.2192, 0.0458		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.6.4.1
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	40	40	17	16		
Mean	0.15879	-0.08046	0.13625	-0.07028		
(SD)	(0.054262)	(0.090588)	(0.054644)	(0.070056)		
Median	0.15340	-0.08090	0.12710	-0.06795		
Min, Max	0.0708, 0.2932	-0.3880, 0.0695	0.0421, 0.2625	-0.2533, 0.0531		
Week 24						
n	42	42	14	14		
Mean	0.14811	-0.08350	0.16635	-0.07383		
(SD)	(0.060436)	(0.077952)	(0.049914)	(0.105340)		
Median	0.13625	-0.06355	0.15850	-0.06400		
Min, Max	0.0351, 0.3700	-0.3735, 0.0452	0.1016, 0.2996	-0.2964, 0.0902		
Week 28						
n	39	39	14	13	53	52
Mean	0.15072	-0.08198	0.13604	-0.07709	0.14684	-0.08076
(SD)	(0.065546)	(0.063844)	(0.027462)	(0.072978)	(0.058059)	(0.065534)
Median	0.14080	-0.08090	0.13150	-0.06830	0.13980	-0.07230
Min, Max	0.0533, 0.4700	-0.2643, 0.0050	0.0968, 0.1874	-0.2632, 0.0455	0.0533, 0.4700	-0.2643, 0.0455

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.1
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	40	40	14	13	54	53
Mean	0.16012	-0.07877	0.13925	-0.07108	0.15471	-0.07688
(SD)	(0.056660)	(0.088792)	(0.055436)	(0.089333)	(0.056581)	(0.088124)
Median	0.15350	-0.04865	0.13030	-0.06870	0.14585	-0.05700
Min, Max	0.0526, 0.2757	-0.3290, 0.0531	0.0689, 0.2940	-0.2627, 0.0846	0.0526, 0.2940	-0.3290, 0.0846
Week 36						
n	33	33	16	16	49	49
Mean	0.15668	-0.09014	0.14575	-0.07725	0.15311	-0.08593
(SD)	(0.054900)	(0.082722)	(0.050471)	(0.069762)	(0.053219)	(0.078231)
Median	0.15050	-0.07540	0.14025	-0.06005	0.14870	-0.06960
Min, Max	0.0701, 0.3384	-0.3076, 0.0860	0.0734, 0.2678	-0.2388, 0.0354	0.0701, 0.3384	-0.3076, 0.0860
Week 40						
n	35	35	11	11	46	46
Mean	0.16193	-0.07825	0.16445	-0.07823	0.16253	-0.07824
(SD)	(0.047571)	(0.079938)	(0.090538)	(0.061703)	(0.059435)	(0.075326)
Median	0.15810	-0.07100	0.14240	-0.07300	0.15455	-0.07260
Min, Max	0.0839, 0.2831	-0.2646, 0.0538	0.0567, 0.4052	-0.1973, 0.0382	0.0567, 0.4052	-0.2646, 0.0538

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.1
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	34	34	14	14	48	48
Mean	0.16085	-0.08499	0.17404	-0.06161	0.16470	-0.07818
(SD)	(0.059634)	(0.074689)	(0.061554)	(0.082290)	(0.059846)	(0.076845)
Median	0.15305	-0.06210	0.16885	-0.06010	0.15595	-0.06210
Min, Max	0.0675, 0.3216	-0.3035, 0.0464	0.1018, 0.3040	-0.2264, 0.0721	0.0675, 0.3216	-0.3035, 0.0721
Week 48						
n	29	29	12	11	41	40
Mean	0.16420	-0.08007	0.14967	-0.10635	0.15994	-0.08730
(SD)	(0.050516)	(0.065770)	(0.053001)	(0.088758)	(0.051026)	(0.072574)
Median	0.16200	-0.07140	0.13460	-0.09430	0.14770	-0.07390
Min, Max	0.0903, 0.2753	-0.2917, 0.0105	0.0788, 0.2721	-0.3072, 0.0281	0.0788, 0.2753	-0.3072, 0.0281
Week 52						
n	23	23	10	9	33	32
Mean	0.15322	-0.07619	0.13876	-0.07862	0.14884	-0.07687
(SD)	(0.043857)	(0.064157)	(0.069430)	(0.050686)	(0.052189)	(0.059878)
Median	0.14720	-0.06620	0.11625	-0.07340	0.14340	-0.07185
Min, Max	0.0764, 0.2693	-0.2386, -0.0029	0.0600, 0.3085	-0.1428, 0.0128	0.0600, 0.3085	-0.2386, 0.0128

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB
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Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.1
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	29	29	10	9	39	38
Mean	0.15770	-0.09008	0.15558	-0.10624	0.15716	-0.09391
(SD)	(0.057612)	(0.079120)	(0.054648)	(0.071261)	(0.056159)	(0.076706)
Median	0.14000	-0.08920	0.14040	-0.07790	0.14000	-0.08355
Min, Max	0.0439, 0.2662	-0.3276, -0.0079	0.0800, 0.2705	-0.2573, -0.0427	0.0439, 0.2705	-0.3276, -0.0079
Week 60						
n	22	22	11	11	33	33
Mean	0.16679	-0.09227	0.15244	-0.05478	0.16201	-0.07977
(SD)	(0.053423)	(0.078428)	(0.057652)	(0.057849)	(0.054395)	(0.073514)
Median	0.15580	-0.07180	0.13640	-0.05710	0.15160	-0.05810
Min, Max	0.0906, 0.2700	-0.2899, -0.0025	0.1079, 0.3121	-0.1504, 0.0550	0.0906, 0.3121	-0.2899, 0.0550
Week 64						
n	19	19	11	11	30	30
Mean	0.15452	-0.09777	0.14415	-0.08106	0.15071	-0.09165
(SD)	(0.078992)	(0.082647)	(0.061954)	(0.084485)	(0.072266)	(0.082268)
Median	0.13740	-0.10150	0.13980	-0.07240	0.13805	-0.08110
Min, Max	0.0500, 0.3500	-0.2752, 0.0354	0.0500, 0.2906	-0.2974, 0.0185	0.0500, 0.3500	-0.2974, 0.0354

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.1
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	15	15	9	9	24	24
Mean	0.14755	-0.09667	0.14049	-0.06889	0.14490	-0.08625
(SD)	(0.061958)	(0.057227)	(0.073587)	(0.053709)	(0.065057)	(0.056440)
Median	0.13820	-0.09960	0.12480	-0.07140	0.13575	-0.07725
Min, Max	0.0637, 0.2867	-0.2357, -0.0030	0.0500, 0.3011	-0.1771, 0.0206	0.0500, 0.3011	-0.2357, 0.0206
Week 72						
n	14	14	8	8	22	22
Mean	0.17741	-0.09216	0.19115	-0.04615	0.18241	-0.07543
(SD)	(0.085619)	(0.076274)	(0.102715)	(0.060015)	(0.090003)	(0.072906)
Median	0.15040	-0.07310	0.14315	-0.05615	0.14890	-0.07105
Min, Max	0.0932, 0.3833	-0.2697, -0.0002	0.1032, 0.3596	-0.1290, 0.0438	0.0932, 0.3833	-0.2697, 0.0438
Week 80						
n	11	11	7	7	18	18
Mean	0.16843	-0.09076	0.12474	-0.12173	0.15144	-0.10281
(SD)	(0.077230)	(0.077660)	(0.036820)	(0.130231)	(0.066837)	(0.098868)
Median	0.13460	-0.09640	0.12840	-0.06070	0.13395	-0.07855
Min, Max	0.0829, 0.3500	-0.2474, 0.0200	0.0505, 0.1697	-0.3085, 0.0376	0.0505, 0.3500	-0.3085, 0.0376

Baseline is defined as the last non-missing value prior to the first dose of study drug.
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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.1
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	3	3	9	9
Mean	0.15653	-0.11268	0.22537	-0.05853	0.17948	-0.09463
(SD)	(0.051040)	(0.128702)	(0.048097)	(0.088367)	(0.058232)	(0.114183)
Median	0.16970	-0.07825	0.19820	-0.04650	0.19190	-0.06950
Min, Max	0.0788, 0.2155	-0.3612, 0.0073	0.1970, 0.2809	-0.1523, 0.0232	0.0788, 0.2809	-0.3612, 0.0232
Week 96						
n	4	4	4	4	8	8
Mean	0.16990	-0.06693	0.11378	-0.12695	0.14184	-0.09694
(SD)	(0.017241)	(0.041603)	(0.032778)	(0.136454)	(0.038573)	(0.098748)
Median	0.17140	-0.06640	0.12110	-0.07635	0.14530	-0.06855
Min, Max	0.1474, 0.1894	-0.1092, -0.0257	0.0697, 0.1432	-0.3245, -0.0306	0.0697, 0.1894	-0.3245, -0.0257
Week 104						
n	1	1	2	2	3	3
Mean	0.17020	-0.11480	0.13280	-0.05615	0.14527	-0.07570
(SD)	(NA)	(NA)	(0.080327)	(0.001485)	(0.060766)	(0.033878)
Median	0.17020	-0.11480	0.13280	-0.05615	0.17020	-0.05720
Min, Max	0.1702, 0.1702	-0.1148, -0.1148	0.0760, 0.1896	-0.0572, -0.0551	0.0760, 0.1896	-0.1148, -0.0551

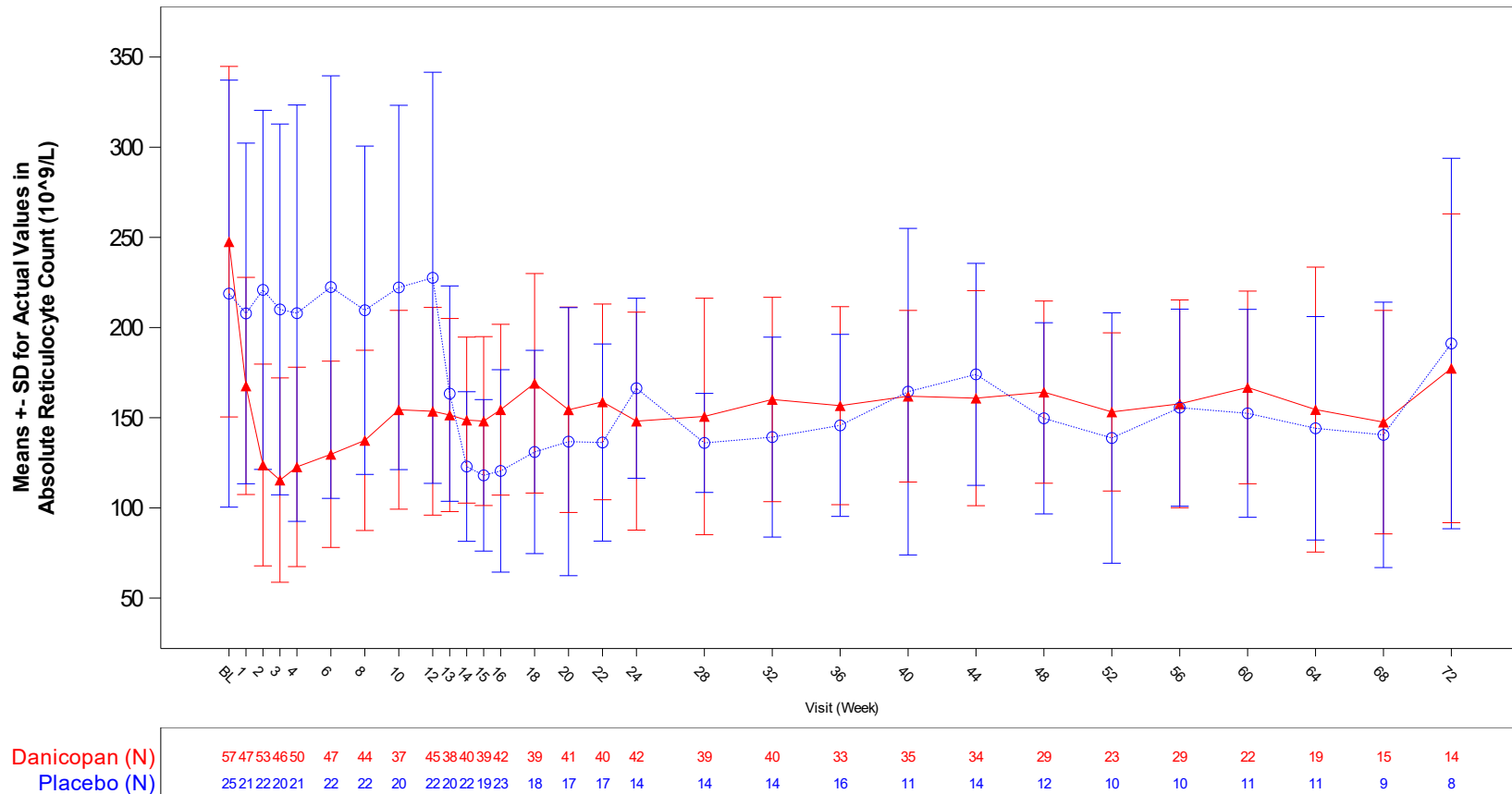
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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Source: ADaM.ADSL, ADaM.ADLB
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Figure GERMANY.2.2.2.3.1
Plots of Mean \pm SD for Actual Values in Absolute Reticulocyte Count ($10^9/L$) During 72 Weeks
Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADLB

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Table GERMANY.2.2.4.1
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-0.0742 (0.00620)	-0.0097 (0.00958)	-0.0645 (0.01126)	<0.0001
	95% CI for LS MEAN	(-0.0866, -0.0618)	(-0.0288, 0.0095)	(-0.0870, -0.0421)	
Week 2	LS Mean (SEM)	-0.1213 (0.00812)	-0.0050 (0.01271)	-0.1163 (0.01494)	<0.0001
	95% CI for LS MEAN	(-0.1374, -0.1051)	(-0.0303, 0.0204)	(-0.1461, -0.0865)	
Week 3	LS Mean (SEM)	-0.1309 (0.00853)	-0.0122 (0.01324)	-0.1187 (0.01562)	<0.0001
	95% CI for LS MEAN	(-0.1479, -0.1139)	(-0.0386, 0.0142)	(-0.1498, -0.0875)	
Week 4	LS Mean (SEM)	-0.1215 (0.00949)	-0.0119 (0.01466)	-0.1096 (0.01734)	<0.0001
	95% CI for LS MEAN	(-0.1404, -0.1026)	(-0.0412, 0.0174)	(-0.1442, -0.0750)	
Week 6	LS Mean (SEM)	-0.1128 (0.00738)	-0.0042 (0.01116)	-0.1086 (0.01322)	<0.0001
	95% CI for LS MEAN	(-0.1276, -0.0981)	(-0.0266, 0.0181)	(-0.1350, -0.0822)	
Week 8	LS Mean (SEM)	-0.1028 (0.00693)	-0.0094 (0.01016)	-0.0934 (0.01211)	<0.0001
	95% CI for LS MEAN	(-0.1166, -0.0890)	(-0.0297, 0.0109)	(-0.1176, -0.0692)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). An Unstructured covariance structure was used.

P-value is from difference of LSM from MMRM model.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.2.4.1
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-0.0923 (0.00754)	-0.0017 (0.01077)	-0.0906 (0.01300)	<0.0001
	95% CI for LS MEAN	(-0.1073, -0.0773)	(-0.0232, 0.0198)	(-0.1166, -0.0647)	
Week 12	LS Mean (SEM)	-0.0931 (0.00819)	-0.0034 (0.01213)	-0.0896 (0.01449)	<0.0001
	95% CI for LS MEAN	(-0.1094, -0.0768)	(-0.0276, 0.0207)	(-0.1185, -0.0608)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). An Unstructured covariance structure was used.

P-value is from difference of LSM from MMRM model.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

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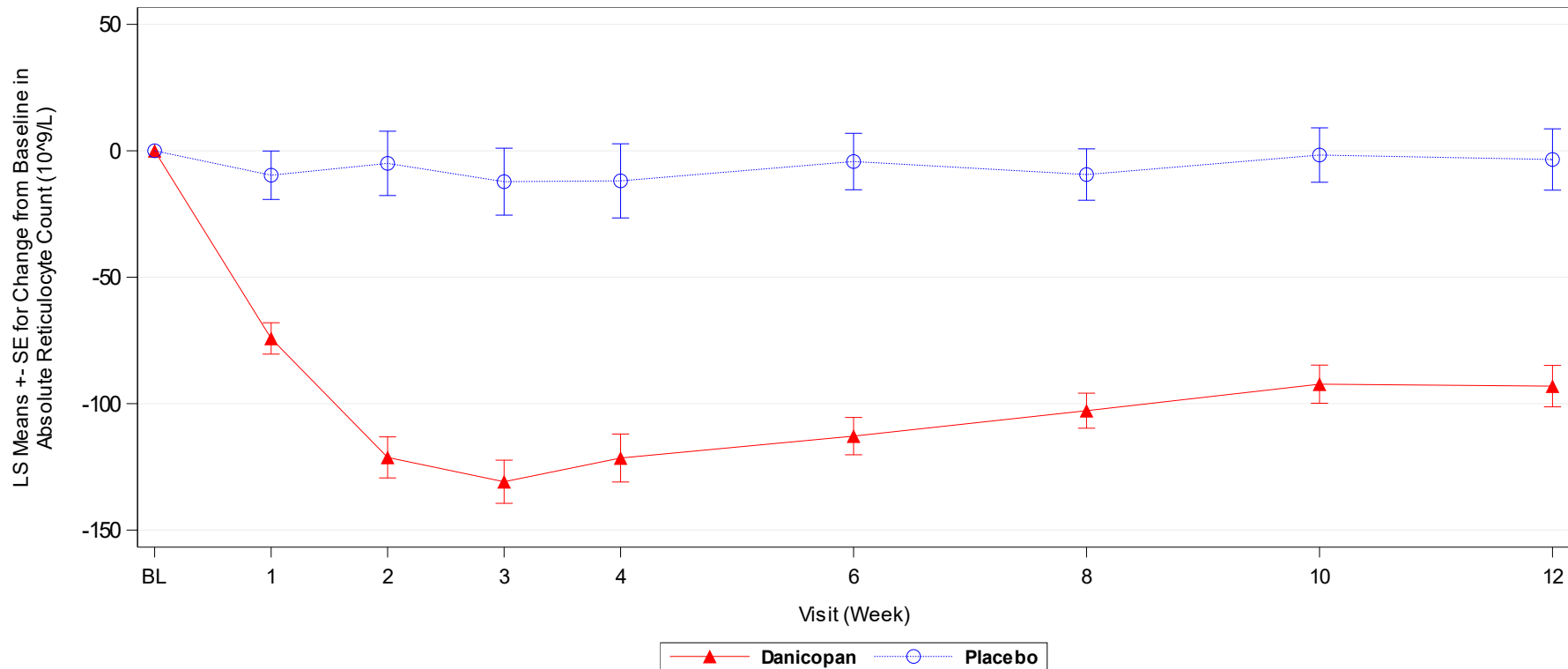
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Figure GERMANY.2.2.2.1.1
Plots of LS Means \pm SE for Change from Baseline in Absolute Reticulocyte Count ($10^9/L$)
During 12-Week of Treatment Period 1
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).
Source: ADaM.ADSL, ADaM.ADLB
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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.4 Veränderung der absoluten Retikulozytenzahl

1.4.1 Subgruppenanalysen – Veränderung der absoluten Retikulozytenzahl gegenüber Baseline

Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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Table GERMANY.2.6.4.2
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean	0.24852		0.19857		0.23447	
(SD)	(0.116277)		(0.047157)		(0.103390)	
Median	0.21130		0.19940		0.20545	
Min, Max	0.1365, 0.5295		0.1300, 0.2672		0.1300, 0.5295	
Week 1						
n	20	20	9	9		
Mean	0.17467	-0.06384	0.18544	-0.01312		
(SD)	(0.062200)	(0.071006)	(0.050859)	(0.024039)		
Median	0.17375	-0.03320	0.21430	-0.01000		
Min, Max	0.0789, 0.3200	-0.2457, 0.0183	0.1015, 0.2382	-0.0473, 0.0165		
Week 2						
n	21	21	8	8		
Mean	0.14681	-0.10682	0.19051	-0.00516		
(SD)	(0.044349)	(0.118375)	(0.048097)	(0.013454)		
Median	0.14060	-0.07490	0.18805	-0.00665		
Min, Max	0.0671, 0.2299	-0.4371, 0.0089	0.1257, 0.2531	-0.0231, 0.0172		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.4.2
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	21	21	7	7		
Mean	0.13571	-0.10495	0.19750	-0.00756		
(SD)	(0.059653)	(0.105031)	(0.042093)	(0.021035)		
Median	0.13450	-0.08980	0.20280	0.00140		
Min, Max	0.0500, 0.2479	-0.4571, 0.0148	0.1409, 0.2633	-0.0478, 0.0128		
Week 4						
n	20	20	8	8		
Mean	0.14435	-0.09991	0.18713	-0.00855		
(SD)	(0.049257)	(0.090593)	(0.064464)	(0.045595)		
Median	0.14755	-0.09215	0.16035	-0.00655		
Min, Max	0.0597, 0.2300	-0.3445, 0.0100	0.1300, 0.3198	-0.0993, 0.0579		
Week 6						
n	21	21	8	8		
Mean	0.14800	-0.08991	0.19143	-0.00425		
(SD)	(0.061740)	(0.072139)	(0.062386)	(0.025425)		
Median	0.14410	-0.07980	0.20610	0.00650		
Min, Max	0.0669, 0.3100	-0.2861, 0.0255	0.1000, 0.2718	-0.0464, 0.0301		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	17	17	9	9		
Mean	0.15726	-0.08062	0.20359	0.00502		
(SD)	(0.060862)	(0.096410)	(0.060738)	(0.037462)		
Median	0.15560	-0.06330	0.19540	-0.00060		
Min, Max	0.0839, 0.3244	-0.2995, 0.0651	0.1000, 0.3000	-0.0397, 0.0764		
Week 10						
n	17	17	7	7		
Mean	0.17015	-0.08509	0.18146	0.00174		
(SD)	(0.056876)	(0.093933)	(0.037407)	(0.022564)		
Median	0.14500	-0.06950	0.17670	0.00290		
Min, Max	0.1107, 0.3238	-0.2859, 0.0645	0.1400, 0.2518	-0.0347, 0.0301		
Week 12						
n	22	22	9	9		
Mean	0.16315	-0.08648	0.19880	0.00023		
(SD)	(0.066929)	(0.103778)	(0.066352)	(0.038044)		
Median	0.14845	-0.05490	0.20750	0.00990		
Min, Max	0.0612, 0.3537	-0.3279, 0.0944	0.1000, 0.3346	-0.0597, 0.0727		

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Table GERMANY.2.6.4.2
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	15	15	6	6		
Mean	0.17039	-0.07251	0.14917	-0.04743		
(SD)	(0.054982)	(0.096454)	(0.034223)	(0.035702)		
Median	0.15610	-0.03570	0.15570	-0.04165		
Min, Max	0.1008, 0.3100	-0.3539, 0.0076	0.0966, 0.1823	-0.0972, -0.0053		
Week 14						
n	18	18	8	8		
Mean	0.15591	-0.08583	0.12819	-0.06180		
(SD)	(0.050336)	(0.092766)	(0.042224)	(0.044696)		
Median	0.15835	-0.05155	0.12505	-0.07385		
Min, Max	0.0733, 0.2720	-0.3537, 0.0100	0.0636, 0.2053	-0.1297, -0.0100		
Week 15						
n	18	18	7	7		
Mean	0.15138	-0.06943	0.11034	-0.08553		
(SD)	(0.043061)	(0.089867)	(0.021637)	(0.037420)		
Median	0.15675	-0.04270	0.11430	-0.08510		
Min, Max	0.0880, 0.2399	-0.3686, 0.0189	0.0835, 0.1467	-0.1382, -0.0400		

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Table GERMANY.2.6.4.2
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	20	20	9	9		
Mean	0.15257	-0.06576	0.11456	-0.08401		
(SD)	(0.040212)	(0.071324)	(0.041592)	(0.034973)		
Median	0.15270	-0.04265	0.10640	-0.08930		
Min, Max	0.0819, 0.2184	-0.3234, -0.0059	0.0516, 0.1970	-0.1377, -0.0353		
Week 18						
n	16	16	7	7		
Mean	0.17859	-0.04989	0.13527	-0.05181		
(SD)	(0.059554)	(0.057644)	(0.038812)	(0.038962)		
Median	0.17525	-0.02660	0.11840	-0.05540		
Min, Max	0.0960, 0.3164	-0.1954, 0.0299	0.0800, 0.1969	-0.1058, 0.0060		
Week 20						
n	17	17	6	6		
Mean	0.16004	-0.07841	0.11472	-0.07455		
(SD)	(0.050566)	(0.079849)	(0.025665)	(0.028151)		
Median	0.15570	-0.05630	0.11480	-0.06550		
Min, Max	0.0976, 0.2900	-0.3087, -0.0063	0.0800, 0.1565	-0.1115, -0.0494		

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Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	17	17	6	6		
Mean	0.16152	-0.07531	0.14040	-0.07173		
(SD)	(0.048702)	(0.094126)	(0.042242)	(0.016977)		
Median	0.17140	-0.03280	0.14140	-0.07575		
Min, Max	0.0835, 0.2537	-0.3880, 0.0127	0.0729, 0.1916	-0.0894, -0.0489		
Week 24						
n	21	21	6	6		
Mean	0.15949	-0.07620	0.16592	-0.04622		
(SD)	(0.068780)	(0.081689)	(0.039452)	(0.044501)		
Median	0.13830	-0.05500	0.15985	-0.04725		
Min, Max	0.0351, 0.3700	-0.3735, 0.0011	0.1159, 0.2273	-0.1058, 0.0261		
Week 28						
n	17	17	5	5	22	22
Mean	0.16162	-0.05891	0.12840	-0.07378	0.15407	-0.06229
(SD)	(0.090728)	(0.033778)	(0.022851)	(0.033119)	(0.081081)	(0.033450)
Median	0.14360	-0.05690	0.12320	-0.05960	0.13580	-0.05820
Min, Max	0.0533, 0.4700	-0.1066, 0.0050	0.1084, 0.1621	-0.1132, -0.0404	0.0533, 0.4700	-0.1132, 0.0050

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	17	17	5	5	22	22
Mean	0.17762	-0.05605	0.11162	-0.07222	0.16262	-0.05972
(SD)	(0.051502)	(0.093673)	(0.030490)	(0.040974)	(0.054768)	(0.083984)
Median	0.17470	-0.02530	0.12990	-0.06870	0.14585	-0.03590
Min, Max	0.1190, 0.2757	-0.3290, 0.0260	0.0689, 0.1386	-0.1373, -0.0352	0.0689, 0.2757	-0.3290, 0.0260
Week 36						
n	16	16	6	6	22	22
Mean	0.16611	-0.08708	0.14680	-0.06343	0.16084	-0.08063
(SD)	(0.046678)	(0.087482)	(0.047289)	(0.019153)	(0.046542)	(0.075300)
Median	0.15970	-0.05415	0.14480	-0.06005	0.15390	-0.06005
Min, Max	0.1098, 0.2563	-0.3076, -0.0093	0.0734, 0.2161	-0.0949, -0.0444	0.0734, 0.2563	-0.3076, -0.0093
Week 40						
n	16	16	4	4	20	20
Mean	0.17347	-0.04368	0.12143	-0.07588	0.16306	-0.05012
(SD)	(0.045543)	(0.064436)	(0.045551)	(0.030733)	(0.049207)	(0.060014)
Median	0.17675	-0.03375	0.13330	-0.08215	0.15910	-0.03830
Min, Max	0.1118, 0.2831	-0.2464, 0.0538	0.0567, 0.1624	-0.1048, -0.0344	0.0567, 0.2831	-0.2464, 0.0538

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	17	17	5	5	22	22
Mean	0.17919	-0.06878	0.16716	-0.04810	0.17645	-0.06408
(SD)	(0.064757)	(0.083922)	(0.038757)	(0.047128)	(0.059227)	(0.076602)
Median	0.16540	-0.04170	0.18180	-0.06560	0.16805	-0.05210
Min, Max	0.0948, 0.3216	-0.3035, 0.0464	0.1239, 0.2061	-0.0854, 0.0321	0.0948, 0.3216	-0.3035, 0.0464
Week 48						
n	14	14	3	3	17	17
Mean	0.16963	-0.06338	0.13847	-0.07500	0.16413	-0.06543
(SD)	(0.048507)	(0.075894)	(0.019398)	(0.029414)	(0.045921)	(0.069346)
Median	0.17860	-0.04565	0.13690	-0.06250	0.15860	-0.05390
Min, Max	0.0903, 0.2552	-0.2917, 0.0105	0.1199, 0.1586	-0.1086, -0.0539	0.0903, 0.2552	-0.2917, 0.0105
Week 52						
n	11	11	3	3	14	14
Mean	0.14339	-0.05015	0.11910	-0.07123	0.13819	-0.05467
(SD)	(0.041928)	(0.037228)	(0.068250)	(0.001882)	(0.046646)	(0.033871)
Median	0.14720	-0.04010	0.10350	-0.07030	0.13710	-0.05610
Min, Max	0.0764, 0.2081	-0.1151, -0.0029	0.0600, 0.1938	-0.0734, -0.0700	0.0600, 0.2081	-0.1151, -0.0029

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	14	14	3	3	17	17
Mean	0.16819	-0.08657	0.11563	-0.07470	0.15891	-0.08448
(SD)	(0.058067)	(0.097143)	(0.032455)	(0.042436)	(0.057426)	(0.088962)
Median	0.18300	-0.04130	0.12340	-0.05040	0.14350	-0.05000
Min, Max	0.0736, 0.2581	-0.3276, -0.0147	0.0800, 0.1435	-0.1237, -0.0500	0.0736, 0.2581	-0.3276, -0.0147
Week 60						
n	12	12	3	3	15	15
Mean	0.19148	-0.08121	0.13050	-0.05983	0.17929	-0.07693
(SD)	(0.052046)	(0.093719)	(0.030703)	(0.040728)	(0.053856)	(0.084950)
Median	0.19150	-0.04895	0.11570	-0.05810	0.16680	-0.04970
Min, Max	0.1061, 0.2700	-0.2899, -0.0025	0.1100, 0.1658	-0.1014, -0.0200	0.1061, 0.2700	-0.2899, -0.0025
Week 64						
n	10	10	3	3	13	13
Mean	0.16788	-0.08165	0.12087	-0.06947	0.15703	-0.07884
(SD)	(0.081173)	(0.082236)	(0.063552)	(0.031548)	(0.077717)	(0.072571)
Median	0.14670	-0.05170	0.13980	-0.08000	0.14340	-0.06560
Min, Max	0.0833, 0.3500	-0.2592, -0.0079	0.0500, 0.1728	-0.0944, -0.0340	0.0500, 0.3500	-0.2592, -0.0079

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.2
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	7	7	3	3	10	10
Mean	0.14609	-0.08617	0.12060	-0.06973	0.13844	-0.08124
(SD)	(0.060275)	(0.044651)	(0.073009)	(0.011193)	(0.061303)	(0.037684)
Median	0.14280	-0.06690	0.11600	-0.07140	0.13805	-0.06915
Min, Max	0.0637, 0.2565	-0.1565, -0.0430	0.0500, 0.1958	-0.0800, -0.0578	0.0500, 0.2565	-0.1565, -0.0430
Week 72						
n	6	6	1	1	7	7
Mean	0.16577	-0.09862	0.13140	-0.04240	0.16086	-0.09059
(SD)	(0.068277)	(0.076610)	(NA)	(NA)	(0.063667)	(0.073092)
Median	0.15040	-0.07310	0.13140	-0.04240	0.14980	-0.07210
Min, Max	0.1078, 0.3000	-0.2295, -0.0287	0.1314, 0.1314	-0.0424, -0.0424	0.1078, 0.3000	-0.2295, -0.0287
Week 80						
n	6	6	1	1	7	7
Mean	0.15973	-0.10410	0.12210	-0.05170	0.15436	-0.09661
(SD)	(0.098224)	(0.051434)	(NA)	(NA)	(0.090787)	(0.050958)
Median	0.12430	-0.11315	0.12210	-0.05170	0.12210	-0.10860
Min, Max	0.0829, 0.3500	-0.1795, -0.0338	0.1221, 0.1221	-0.0517, -0.0517	0.0829, 0.3500	-0.1795, -0.0338

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.2
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	2	2	1	1	3	3
Mean	0.14450	-0.06775	0.19700	0.02320	0.16200	-0.03743
(SD)	(0.043699)	(0.027224)	(NA)	(NA)	(0.043285)	(0.055927)
Median	0.14450	-0.06775	0.19700	0.02320	0.17540	-0.04850
Min, Max	0.1136, 0.1754	-0.0870, -0.0485	0.1970, 0.1970	0.0232, 0.0232	0.1136, 0.1970	-0.0870, 0.0232
Week 96						
n	1	1	1	1	2	2
Mean	0.17080	-0.02570	0.14320	-0.03060	0.15700	-0.02815
(SD)	(NA)	(NA)	(NA)	(NA)	(0.019516)	(0.003465)
Median	0.17080	-0.02570	0.14320	-0.03060	0.15700	-0.02815
Min, Max	0.1708, 0.1708	-0.0257, -0.0257	0.1432, 0.1432	-0.0306, -0.0306	0.1432, 0.1708	-0.0306, -0.0257

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Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.4.2
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		16		50	
Mean	0.24701		0.23021		0.24163	
(SD)	(0.083748)		(0.144409)		(0.105689)	
Median	0.23065		0.18085		0.22200	
Min, Max	0.1094, 0.4400		0.0394, 0.5419		0.0394, 0.5419	
Week 1						
n	27	27	12	11		
Mean	0.16240	-0.08810	0.22462	0.01923		
(SD)	(0.059332)	(0.056782)	(0.116696)	(0.047134)		
Median	0.15690	-0.08250	0.22205	0.02590		
Min, Max	0.0553, 0.2864	-0.2100, -0.0100	0.0410, 0.4444	-0.0975, 0.0929		
Week 2						
n	32	32	14	13		
Mean	0.10869	-0.14045	0.23826	0.00847		
(SD)	(0.058189)	(0.084140)	(0.117792)	(0.039390)		
Median	0.10060	-0.13250	0.22190	0.01610		
Min, Max	0.0388, 0.3474	-0.3609, -0.0253	0.0402, 0.4748	-0.0671, 0.0686		

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Table GERMANY.2.6.4.2
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	25	25	13	13		
Mean	0.09838	-0.13948	0.21680	-0.00210		
(SD)	(0.048899)	(0.080124)	(0.125328)	(0.044296)		
Median	0.08620	-0.13460	0.20830	-0.00850		
Min, Max	0.0400, 0.2506	-0.4000, -0.0291	0.0309, 0.5193	-0.1185, 0.0529		
Week 4						
n	30	30	13	13		
Mean	0.10833	-0.12981	0.22079	-0.00991		
(SD)	(0.055114)	(0.097170)	(0.139008)	(0.048247)		
Median	0.09760	-0.14405	0.20250	0.01120		
Min, Max	0.0326, 0.2846	-0.3350, 0.1083	0.0388, 0.5743	-0.1138, 0.0411		
Week 6						
n	26	26	14	14		
Mean	0.11495	-0.11526	0.24015	0.00273		
(SD)	(0.036765)	(0.077662)	(0.138365)	(0.037961)		
Median	0.11140	-0.11365	0.22040	0.00045		
Min, Max	0.0554, 0.1881	-0.3016, 0.0035	0.0343, 0.5254	-0.0716, 0.0611		

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Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	27	27	13	13		
Mean	0.12494	-0.10292	0.21375	-0.00515		
(SD)	(0.037824)	(0.058217)	(0.109518)	(0.049086)		
Median	0.11900	-0.11130	0.22280	-0.00070		
Min, Max	0.0682, 0.2053	-0.2192, 0.0106	0.0424, 0.4560	-0.0859, 0.0827		
Week 10						
n	20	20	13	13		
Mean	0.14104	-0.10559	0.24418	0.01221		
(SD)	(0.051218)	(0.059361)	(0.118156)	(0.057590)		
Median	0.13525	-0.10495	0.24720	0.01410		
Min, Max	0.0534, 0.2541	-0.2202, 0.0017	0.1180, 0.5817	-0.1417, 0.0855		
Week 12						
n	23	23	13	13		
Mean	0.14441	-0.09742	0.24752	0.00415		
(SD)	(0.046662)	(0.087094)	(0.136935)	(0.058010)		
Median	0.14000	-0.09360	0.25470	-0.00210		
Min, Max	0.0800, 0.2385	-0.3000, 0.0311	0.0383, 0.5375	-0.1279, 0.0948		

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Table GERMANY.2.6.4.2
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	23	23	14	13		
Mean	0.13917	-0.10054	0.16946	-0.03487		
(SD)	(0.049933)	(0.090147)	(0.068013)	(0.089649)		
Median	0.13710	-0.10350	0.17200	-0.02220		
Min, Max	0.0495, 0.2536	-0.2800, 0.0460	0.0291, 0.3126	-0.2200, 0.0841		
Week 14						
n	22	22	14	14		
Mean	0.14277	-0.10723	0.11994	-0.09374		
(SD)	(0.042426)	(0.085333)	(0.042343)	(0.120135)		
Median	0.13350	-0.11020	0.12475	-0.07475		
Min, Max	0.0642, 0.2018	-0.2621, 0.0018	0.0241, 0.1988	-0.3603, 0.0501		
Week 15						
n	21	21	12	11		
Mean	0.14536	-0.09504	0.12255	-0.08820		
(SD)	(0.050709)	(0.089075)	(0.050715)	(0.097670)		
Median	0.12820	-0.09210	0.12240	-0.08680		
Min, Max	0.0736, 0.2517	-0.2845, 0.0437	0.0328, 0.2208	-0.2673, 0.0599		

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Post-hoc Analysen Studie ALPHA

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Table GERMANY.2.6.4.2
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	22	22	14	13		
Mean	0.15616	-0.09825	0.12432	-0.09278		
(SD)	(0.053920)	(0.094637)	(0.064997)	(0.098160)		
Median	0.15290	-0.09110	0.10910	-0.08340		
Min, Max	0.0832, 0.2986	-0.2811, 0.0749	0.0315, 0.3029	-0.2719, 0.0639		
Week 18						
n	23	23	11	10		
Mean	0.16249	-0.07833	0.12829	-0.09354		
(SD)	(0.062193)	(0.084094)	(0.066886)	(0.090896)		
Median	0.15300	-0.08150	0.12050	-0.09755		
Min, Max	0.0761, 0.2993	-0.2201, 0.0800	0.0461, 0.2377	-0.2256, 0.0396		
Week 20						
n	24	24	11	10		
Mean	0.15042	-0.10012	0.14875	-0.08670		
(SD)	(0.061815)	(0.079651)	(0.089771)	(0.076222)		
Median	0.14145	-0.11425	0.12440	-0.08280		
Min, Max	0.0538, 0.2949	-0.2686, 0.0712	0.0362, 0.3278	-0.2192, 0.0458		

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Post-hoc Analysen Studie ALPHA

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Table GERMANY.2.6.4.2
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Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	23	23	11	10		
Mean	0.15677	-0.08426	0.13399	-0.06941		
(SD)	(0.059030)	(0.089825)	(0.062205)	(0.089540)		
Median	0.14570	-0.08970	0.11970	-0.05925		
Min, Max	0.0708, 0.2932	-0.2762, 0.0695	0.0421, 0.2625	-0.2533, 0.0531		
Week 24						
n	21	21	8	8		
Mean	0.13673	-0.09080	0.16668	-0.09454		
(SD)	(0.049848)	(0.075313)	(0.059287)	(0.134349)		
Median	0.13330	-0.07490	0.15850	-0.08635		
Min, Max	0.0504, 0.2408	-0.2724, 0.0452	0.1016, 0.2996	-0.2964, 0.0902		
Week 28						
n	22	22	9	8	31	30
Mean	0.14230	-0.09980	0.14028	-0.07916	0.14171	-0.09430
(SD)	(0.036497)	(0.075782)	(0.030129)	(0.092143)	(0.034284)	(0.079336)
Median	0.13810	-0.09830	0.14960	-0.07230	0.14080	-0.08640
Min, Max	0.0843, 0.2376	-0.2643, -0.0006	0.0968, 0.1874	-0.2632, 0.0455	0.0843, 0.2376	-0.2643, 0.0455

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ALXN2040-PNH-301

Table GERMANY.2.6.4.2
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	23	23	9	8	32	31
Mean	0.14718	-0.09557	0.15460	-0.07036	0.14927	-0.08906
(SD)	(0.057882)	(0.083071)	(0.061538)	(0.112782)	(0.058020)	(0.090300)
Median	0.14990	-0.09170	0.13310	-0.06545	0.14480	-0.08510
Min, Max	0.0526, 0.2538	-0.3003, 0.0531	0.0977, 0.2940	-0.2627, 0.0846	0.0526, 0.2940	-0.3003, 0.0846
Week 36						
n	17	17	10	10	27	27
Mean	0.14782	-0.09302	0.14512	-0.08554	0.14682	-0.09025
(SD)	(0.061751)	(0.080578)	(0.054791)	(0.087771)	(0.058202)	(0.081705)
Median	0.15000	-0.09960	0.13520	-0.06025	0.14210	-0.07850
Min, Max	0.0701, 0.3384	-0.2239, 0.0860	0.0928, 0.2678	-0.2388, 0.0354	0.0701, 0.3384	-0.2388, 0.0860
Week 40						
n	19	19	7	7	26	26
Mean	0.15222	-0.10736	0.18904	-0.07957	0.16213	-0.09988
(SD)	(0.048250)	(0.081564)	(0.103367)	(0.076598)	(0.067216)	(0.079726)
Median	0.15330	-0.09430	0.14780	-0.07300	0.15055	-0.08905
Min, Max	0.0839, 0.2358	-0.2646, 0.0200	0.1109, 0.4052	-0.1973, 0.0382	0.0839, 0.4052	-0.2646, 0.0382

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB
Run Date: 2024-04-26T15:53:40

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.2
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	17	17	9	9	26	26
Mean	0.14252	-0.10121	0.17787	-0.06912	0.15475	-0.09010
(SD)	(0.049261)	(0.062482)	(0.073211)	(0.098569)	(0.059685)	(0.076485)
Median	0.13840	-0.09830	0.16620	-0.05460	0.14495	-0.08545
Min, Max	0.0675, 0.2299	-0.2311, -0.0200	0.1018, 0.3040	-0.2264, 0.0721	0.0675, 0.3040	-0.2311, 0.0721
Week 48						
n	15	15	9	8	24	23
Mean	0.15913	-0.09564	0.15340	-0.11811	0.15698	-0.10346
(SD)	(0.053496)	(0.052580)	(0.060875)	(0.102116)	(0.055127)	(0.072090)
Median	0.12930	-0.10430	0.13230	-0.09965	0.13080	-0.10430
Min, Max	0.1051, 0.2753	-0.2018, -0.0200	0.0788, 0.2721	-0.3072, 0.0281	0.0788, 0.2753	-0.3072, 0.0281
Week 52						
n	12	12	7	6	19	18
Mean	0.16223	-0.10005	0.14719	-0.08232	0.15668	-0.09414
(SD)	(0.045427)	(0.075322)	(0.073499)	(0.063718)	(0.055833)	(0.070279)
Median	0.15950	-0.10730	0.11700	-0.10200	0.14340	-0.10730
Min, Max	0.0871, 0.2693	-0.2386, -0.0077	0.0938, 0.3085	-0.1428, 0.0128	0.0871, 0.3085	-0.2386, 0.0128

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.2
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	15	15	7	6	22	21
Mean	0.14791	-0.09335	0.17270	-0.12202	0.15580	-0.10154
(SD)	(0.057400)	(0.061102)	(0.054668)	(0.080680)	(0.056480)	(0.066459)
Median	0.12850	-0.09370	0.16490	-0.10610	0.13420	-0.09370
Min, Max	0.0439, 0.2662	-0.2592, -0.0079	0.1240, 0.2705	-0.2573, -0.0427	0.0439, 0.2705	-0.2592, -0.0079
Week 60						
n	10	10	8	8	18	18
Mean	0.13716	-0.10554	0.16066	-0.05289	0.14761	-0.08214
(SD)	(0.039499)	(0.057081)	(0.064771)	(0.065511)	(0.051940)	(0.064938)
Median	0.13370	-0.10070	0.14460	-0.05465	0.13985	-0.08770
Min, Max	0.0906, 0.2059	-0.2387, -0.0330	0.1079, 0.3121	-0.1504, 0.0550	0.0906, 0.3121	-0.2387, 0.0550
Week 64						
n	9	9	8	8	17	17
Mean	0.13967	-0.11569	0.15288	-0.08541	0.14588	-0.10144
(SD)	(0.078456)	(0.084110)	(0.063324)	(0.099163)	(0.069844)	(0.089899)
Median	0.12570	-0.11640	0.13815	-0.06735	0.12570	-0.10150
Min, Max	0.0500, 0.3251	-0.2752, 0.0354	0.0925, 0.2906	-0.2974, 0.0185	0.0500, 0.3251	-0.2974, 0.0354

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.2
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	8	8	6	6	14	14
Mean	0.14884	-0.10585	0.15043	-0.06847	0.14952	-0.08983
(SD)	(0.067525)	(0.068076)	(0.078588)	(0.067563)	(0.069507)	(0.067968)
Median	0.13165	-0.10440	0.13570	-0.06390	0.13165	-0.08940
Min, Max	0.0815, 0.2867	-0.2357, -0.0030	0.0861, 0.3011	-0.1771, 0.0206	0.0815, 0.3011	-0.2357, 0.0206
Week 72						
n	8	8	7	7	15	15
Mean	0.18615	-0.08733	0.19969	-0.04669	0.19247	-0.06836
(SD)	(0.100402)	(0.080929)	(0.107837)	(0.064803)	(0.100364)	(0.074263)
Median	0.14295	-0.07415	0.14800	-0.06990	0.14800	-0.07000
Min, Max	0.0932, 0.3833	-0.2697, -0.0002	0.1032, 0.3596	-0.1290, 0.0438	0.0932, 0.3833	-0.2697, 0.0438
Week 80						
n	5	5	6	6	11	11
Mean	0.17886	-0.07476	0.12518	-0.13340	0.14958	-0.10675
(SD)	(0.051007)	(0.105754)	(0.040314)	(0.138593)	(0.051372)	(0.122537)
Median	0.17590	-0.04130	0.13470	-0.08850	0.14100	-0.06070
Min, Max	0.1239, 0.2399	-0.2474, 0.0200	0.0505, 0.1697	-0.3085, 0.0376	0.0505, 0.2399	-0.3085, 0.0376

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.2
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	4	4	2	2	6	6
Mean	0.16255	-0.13515	0.23955	-0.09940	0.18822	-0.12323
(SD)	(0.059669)	(0.159188)	(0.058478)	(0.074812)	(0.066342)	(0.129092)
Median	0.17795	-0.09335	0.23955	-0.09940	0.19505	-0.09335
Min, Max	0.0788, 0.2155	-0.3612, 0.0073	0.1982, 0.2809	-0.1523, -0.0465	0.0788, 0.2809	-0.3612, 0.0073
Week 96						
n	3	3	3	3	6	6
Mean	0.16960	-0.08067	0.10397	-0.15907	0.13678	-0.11987
(SD)	(0.021103)	(0.038252)	(0.032162)	(0.147447)	(0.043408)	(0.105478)
Median	0.17200	-0.09560	0.10870	-0.11120	0.14045	-0.10240
Min, Max	0.1474, 0.1894	-0.1092, -0.0372	0.0697, 0.1335	-0.3245, -0.0415	0.0697, 0.1894	-0.3245, -0.0372
Week 104						
n	1	1	2	2	3	3
Mean	0.17020	-0.11480	0.13280	-0.05615	0.14527	-0.07570
(SD)	(NA)	(NA)	(0.080327)	(0.001485)	(0.060766)	(0.033878)
Median	0.17020	-0.11480	0.13280	-0.05615	0.17020	-0.05720
Min, Max	0.1702, 0.1702	-0.1148, -0.1148	0.0760, 0.1896	-0.0572, -0.0551	0.0760, 0.1896	-0.1148, -0.0551

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.3

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	41		19		60	
Mean	0.25996		0.22442		0.24871	
(SD)	(0.109795)		(0.131230)		(0.117067)	
Median	0.22370		0.18000		0.21145	
Min, Max	0.1094, 0.5295		0.0394, 0.5419		0.0394, 0.5419	
Week 1						
n	33	33	16	15		
Mean	0.17427	-0.08338	0.21683	0.00533		
(SD)	(0.064836)	(0.069206)	(0.102283)	(0.044438)		
Median	0.17100	-0.07800	0.21845	0.00380		
Min, Max	0.0553, 0.3200	-0.2457, 0.0100	0.0410, 0.4444	-0.0975, 0.0929		
Week 2						
n	39	39	17	16		
Mean	0.12760	-0.13570	0.22503	0.00121		
(SD)	(0.059124)	(0.112153)	(0.107268)	(0.032634)		
Median	0.11970	-0.10850	0.20630	-0.00400		
Min, Max	0.0388, 0.3474	-0.4371, 0.0089	0.0402, 0.4748	-0.0671, 0.0686		

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.3

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	32	32	16	16		
Mean	0.12078	-0.12829	0.20488	-0.00927		
(SD)	(0.062446)	(0.108589)	(0.107960)	(0.038683)		
Median	0.10665	-0.09965	0.19755	-0.00960		
Min, Max	0.0400, 0.2506	-0.4571, 0.0148	0.0309, 0.5193	-0.1185, 0.0529		
Week 4						
n	36	36	17	17		
Mean	0.12725	-0.12206	0.21529	-0.01439		
(SD)	(0.055437)	(0.106706)	(0.126003)	(0.049106)		
Median	0.11570	-0.11875	0.18430	-0.00060		
Min, Max	0.0326, 0.2846	-0.3445, 0.1083	0.0388, 0.5743	-0.1138, 0.0579		
Week 6						
n	34	34	17	17		
Mean	0.13331	-0.10856	0.23066	0.00098		
(SD)	(0.054743)	(0.084731)	(0.120643)	(0.033361)		
Median	0.11640	-0.09510	0.22020	0.00430		
Min, Max	0.0554, 0.3100	-0.3016, 0.0255	0.0343, 0.5254	-0.0716, 0.0611		

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Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.3

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	29	29	16	16		
Mean	0.13382	-0.10580	0.21425	0.00010		
(SD)	(0.041939)	(0.078558)	(0.100883)	(0.047273)		
Median	0.12370	-0.07590	0.20375	0.00040		
Min, Max	0.0682, 0.2300	-0.2995, 0.0106	0.0424, 0.4560	-0.0859, 0.0827		
Week 10						
n	27	27	14	14		
Mean	0.15191	-0.10550	0.23089	0.01179		
(SD)	(0.048932)	(0.079391)	(0.114799)	(0.054483)		
Median	0.14230	-0.07330	0.19670	0.01395		
Min, Max	0.0534, 0.2299	-0.2859, 0.0068	0.1180, 0.5817	-0.1417, 0.0855		
Week 12						
n	34	34	16	16		
Mean	0.15050	-0.10529	0.23414	0.00011		
(SD)	(0.051635)	(0.097658)	(0.128418)	(0.054208)		
Median	0.14060	-0.08445	0.21430	-0.00325		
Min, Max	0.0612, 0.2900	-0.3279, 0.0085	0.0383, 0.5375	-0.1279, 0.0948		

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.3

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	30	30	15	14		
Mean	0.15022	-0.09427	0.17228	-0.02463		
(SD)	(0.054024)	(0.099265)	(0.064733)	(0.078469)		
Median	0.15030	-0.06650	0.18230	-0.01625		
Min, Max	0.0495, 0.3100	-0.3539, 0.0460	0.0291, 0.3126	-0.2200, 0.0841		
Week 14						
n	29	29	16	16		
Mean	0.15230	-0.10253	0.12534	-0.08122		
(SD)	(0.047236)	(0.100326)	(0.040027)	(0.110073)		
Median	0.15340	-0.07040	0.13055	-0.06760		
Min, Max	0.0733, 0.2720	-0.3537, 0.0100	0.0241, 0.1988	-0.3603, 0.0501		
Week 15						
n	27	27	14	13		
Mean	0.14744	-0.08683	0.12713	-0.07496		
(SD)	(0.046560)	(0.101355)	(0.044856)	(0.081100)		
Median	0.14320	-0.05440	0.12590	-0.05740		
Min, Max	0.0736, 0.2437	-0.3686, 0.0437	0.0328, 0.2208	-0.2673, 0.0599		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.3

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	29	29	17	16		
Mean	0.15163	-0.09214	0.13292	-0.07923		
(SD)	(0.047224)	(0.095931)	(0.059854)	(0.082136)		
Median	0.14850	-0.05570	0.12830	-0.07050		
Min, Max	0.0832, 0.2986	-0.3234, 0.0749	0.0315, 0.3029	-0.2719, 0.0639		
Week 18						
n	28	28	12	11		
Mean	0.17186	-0.06862	0.14068	-0.06925		
(SD)	(0.064050)	(0.081510)	(0.064251)	(0.081923)		
Median	0.16450	-0.04585	0.13215	-0.05950		
Min, Max	0.0761, 0.3164	-0.2201, 0.0800	0.0461, 0.2377	-0.2256, 0.0396		
Week 20						
n	31	31	12	11		
Mean	0.16095	-0.09339	0.15147	-0.08543		
(SD)	(0.061436)	(0.087681)	(0.084471)	(0.073255)		
Median	0.15570	-0.06700	0.12500	-0.07850		
Min, Max	0.0538, 0.2949	-0.3087, 0.0712	0.0362, 0.3278	-0.2192, 0.0458		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.3

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	28	28	13	12		
Mean	0.16227	-0.08315	0.14362	-0.06851		
(SD)	(0.050290)	(0.101247)	(0.058459)	(0.081131)		
Median	0.16000	-0.06115	0.12710	-0.05925		
Min, Max	0.0727, 0.2932	-0.3880, 0.0695	0.0421, 0.2625	-0.2533, 0.0531		
Week 24						
n	30	30	10	10		
Mean	0.14816	-0.08826	0.17917	-0.08192		
(SD)	(0.064045)	(0.083907)	(0.051066)	(0.121190)		
Median	0.13745	-0.05690	0.16590	-0.05665		
Min, Max	0.0351, 0.3700	-0.3735, 0.0011	0.1285, 0.2996	-0.2964, 0.0902		
Week 28						
n	29	29	10	9	39	38
Mean	0.15705	-0.08047	0.14344	-0.08008	0.15356	-0.08038
(SD)	(0.071252)	(0.069235)	(0.028299)	(0.087175)	(0.062983)	(0.072599)
Median	0.14570	-0.05950	0.14965	-0.07630	0.14960	-0.06195
Min, Max	0.0533, 0.4700	-0.2643, 0.0050	0.0968, 0.1874	-0.2632, 0.0455	0.0533, 0.4700	-0.2643, 0.0455

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.3

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	29	29	10	9	39	38
Mean	0.16085	-0.08473	0.15510	-0.07362	0.15938	-0.08210
(SD)	(0.058964)	(0.100725)	(0.056833)	(0.108774)	(0.057735)	(0.101286)
Median	0.15600	-0.04560	0.13585	-0.05810	0.14990	-0.04865
Min, Max	0.0526, 0.2757	-0.3290, 0.0531	0.0977, 0.2940	-0.2627, 0.0846	0.0526, 0.2940	-0.3290, 0.0846
Week 36						
n	21	21	13	13	34	34
Mean	0.15873	-0.10258	0.15284	-0.08086	0.15648	-0.09428
(SD)	(0.044018)	(0.086031)	(0.051701)	(0.077342)	(0.046419)	(0.082314)
Median	0.15050	-0.07850	0.14870	-0.06160	0.15025	-0.07170
Min, Max	0.0701, 0.2563	-0.3076, -0.0093	0.0928, 0.2678	-0.2388, 0.0354	0.0701, 0.2678	-0.3076, 0.0354
Week 40						
n	24	24	8	8	32	32
Mean	0.16275	-0.08365	0.18755	-0.07990	0.16895	-0.08271
(SD)	(0.047854)	(0.093746)	(0.094962)	(0.073066)	(0.062083)	(0.087912)
Median	0.16410	-0.05490	0.15210	-0.08065	0.15725	-0.06395
Min, Max	0.0839, 0.2831	-0.2646, 0.0538	0.1109, 0.4052	-0.1973, 0.0382	0.0839, 0.4052	-0.2646, 0.0538

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.3

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	23	23	12	12	35	35
Mean	0.16467	-0.09038	0.18213	-0.06104	0.17066	-0.08032
(SD)	(0.059410)	(0.083287)	(0.063067)	(0.089334)	(0.060343)	(0.085265)
Median	0.15460	-0.05630	0.17665	-0.05585	0.16620	-0.05630
Min, Max	0.0675, 0.3216	-0.3035, -0.0066	0.1018, 0.3040	-0.2264, 0.0721	0.0675, 0.3216	-0.3035, 0.0721
Week 48						
n	21	21	10	9	31	30
Mean	0.17315	-0.07893	0.15443	-0.11561	0.16711	-0.08993
(SD)	(0.051719)	(0.069256)	(0.057052)	(0.096520)	(0.053281)	(0.078550)
Median	0.17290	-0.05550	0.14280	-0.10500	0.15860	-0.08135
Min, Max	0.1051, 0.2753	-0.2917, 0.0006	0.0788, 0.2721	-0.3072, 0.0281	0.0788, 0.2753	-0.3072, 0.0281
Week 52						
n	15	15	9	8	24	23
Mean	0.15903	-0.07339	0.14751	-0.07970	0.15471	-0.07558
(SD)	(0.045815)	(0.072855)	(0.067540)	(0.054076)	(0.053821)	(0.065708)
Median	0.14820	-0.04600	0.11700	-0.07635	0.14530	-0.07030
Min, Max	0.0871, 0.2693	-0.2386, -0.0029	0.0938, 0.3085	-0.1428, 0.0128	0.0871, 0.3085	-0.2386, 0.0128

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

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ALXN2040-PNH-301

Table GERMANY.2.6.4.3

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< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	21	21	8	7	29	28
Mean	0.15496	-0.09874	0.16891	-0.12129	0.15881	-0.10438
(SD)	(0.061194)	(0.089247)	(0.051792)	(0.074692)	(0.058186)	(0.085080)
Median	0.14000	-0.09180	0.15420	-0.12370	0.14350	-0.09275
Min, Max	0.0439, 0.2662	-0.3276, -0.0079	0.1234, 0.2705	-0.2573, -0.0427	0.0439, 0.2705	-0.3276, -0.0079
Week 60						
n	13	13	9	9	22	22
Mean	0.17120	-0.10865	0.16024	-0.05839	0.16672	-0.08809
(SD)	(0.057077)	(0.092983)	(0.061352)	(0.063376)	(0.057671)	(0.084323)
Median	0.15160	-0.06450	0.15280	-0.05810	0.15220	-0.06130
Min, Max	0.0906, 0.2700	-0.2899, -0.0191	0.1079, 0.3121	-0.1504, 0.0550	0.0906, 0.3121	-0.2899, 0.0550
Week 64						
n	13	13	9	9	22	22
Mean	0.16472	-0.09868	0.15848	-0.08214	0.16216	-0.09191
(SD)	(0.091416)	(0.095304)	(0.057507)	(0.094400)	(0.077750)	(0.093028)
Median	0.14340	-0.08220	0.15360	-0.06230	0.14670	-0.07225
Min, Max	0.0500, 0.3500	-0.2752, 0.0354	0.0925, 0.2906	-0.2974, 0.0185	0.0500, 0.3500	-0.2974, 0.0354

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

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Table GERMANY.2.6.4.3

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< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	10	10	8	8	18	18
Mean	0.15367	-0.09855	0.15180	-0.06750	0.15284	-0.08475
(SD)	(0.074041)	(0.066158)	(0.069805)	(0.057245)	(0.070069)	(0.062598)
Median	0.13805	-0.08755	0.13570	-0.06460	0.13805	-0.07295
Min, Max	0.0637, 0.2867	-0.2357, -0.0030	0.0861, 0.3011	-0.1771, 0.0206	0.0637, 0.3011	-0.2357, 0.0206
Week 72						
n	10	10	8	8	18	18
Mean	0.17939	-0.10504	0.19115	-0.04615	0.18462	-0.07887
(SD)	(0.093101)	(0.084842)	(0.102715)	(0.060015)	(0.094706)	(0.078744)
Median	0.15345	-0.07415	0.14315	-0.05615	0.14950	-0.06995
Min, Max	0.0932, 0.3833	-0.2697, -0.0287	0.1032, 0.3596	-0.1290, 0.0438	0.0932, 0.3833	-0.2697, 0.0438
Week 80						
n	8	8	7	7	15	15
Mean	0.19133	-0.08446	0.12474	-0.12173	0.16025	-0.10185
(SD)	(0.078676)	(0.091743)	(0.036820)	(0.130231)	(0.069701)	(0.108846)
Median	0.17505	-0.05060	0.12840	-0.06070	0.14100	-0.06030
Min, Max	0.1027, 0.3500	-0.2474, 0.0200	0.0505, 0.1697	-0.3085, 0.0376	0.0505, 0.3500	-0.3085, 0.0376

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Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.3

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< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	3	3	3	3	6	6
Mean	0.14490	-0.15703	0.22537	-0.05853	0.18513	-0.10778
(SD)	(0.058920)	(0.187452)	(0.048097)	(0.088367)	(0.065241)	(0.141737)
Median	0.16400	-0.11720	0.19820	-0.04650	0.19445	-0.08185
Min, Max	0.0788, 0.1919	-0.3612, 0.0073	0.1970, 0.2809	-0.1523, 0.0232	0.0788, 0.2809	-0.3612, 0.0232
Week 96						
n	3	3	4	4	7	7
Mean	0.16340	-0.05737	0.11378	-0.12695	0.13504	-0.09713
(SD)	(0.013869)	(0.045256)	(0.032778)	(0.136454)	(0.036124)	(0.106658)
Median	0.17080	-0.03720	0.12110	-0.07635	0.14320	-0.04150
Min, Max	0.1474, 0.1720	-0.1092, -0.0257	0.0697, 0.1432	-0.3245, -0.0306	0.0697, 0.1720	-0.3245, -0.0257
Week 104						
n	0	0	2	2	2	2
Mean			0.13280	-0.05615	0.13280	-0.05615
(SD)			(0.080327)	(0.001485)	(0.080327)	(0.001485)
Median			0.13280	-0.05615	0.13280	-0.05615
Min, Max			0.0760, 0.1896	-0.0572, -0.0551	0.0760, 0.1896	-0.0572, -0.0551

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.3

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean	0.21599		0.20107		0.21192	
(SD)	(0.040397)		(0.069131)		(0.048475)	
Median	0.21580		0.19055		0.20995	
Min, Max	0.1200, 0.2850		0.1300, 0.3248		0.1200, 0.3248	
Week 1						
n	14	14	5	5		
Mean	0.15196	-0.06458	0.17902	0.00270		
(SD)	(0.045870)	(0.047758)	(0.063266)	(0.032968)		
Median	0.15265	-0.07925	0.21430	0.01490		
Min, Max	0.0976, 0.2400	-0.1305, 0.0183	0.1015, 0.2382	-0.0473, 0.0394		
Week 2						
n	14	14	5	5		
Mean	0.11319	-0.10325	0.20684	0.00990		
(SD)	(0.046199)	(0.044061)	(0.075485)	(0.033702)		
Median	0.10015	-0.09845	0.21660	0.01000		
Min, Max	0.0419, 0.2075	-0.1904, -0.0449	0.1257, 0.3083	-0.0231, 0.0619		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.3

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	14	14	4	4		
Mean	0.10317	-0.11326	0.23070	0.01703		
(SD)	(0.039766)	(0.039782)	(0.089156)	(0.023650)		
Median	0.09680	-0.11575	0.21440	0.01585		
Min, Max	0.0564, 0.1782	-0.1955, -0.0600	0.1409, 0.3531	-0.0079, 0.0443		
Week 4						
n	14	14	4	4		
Mean	0.11114	-0.10701	0.17685	0.01188		
(SD)	(0.055169)	(0.055254)	(0.050885)	(0.023643)		
Median	0.09910	-0.11065	0.17730	0.00975		
Min, Max	0.0525, 0.2345	-0.1973, -0.0179	0.1300, 0.2228	-0.0131, 0.0411		
Week 6						
n	13	13	5	5		
Mean	0.12033	-0.09183	0.19446	-0.00248		
(SD)	(0.043045)	(0.043565)	(0.112110)	(0.037593)		
Median	0.12150	-0.08170	0.18670	0.00500		
Min, Max	0.0646, 0.1794	-0.1870, -0.0380	0.1000, 0.3751	-0.0464, 0.0503		

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.3

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	15	15	6	6		
Mean	0.14440	-0.07208	0.19718	-0.00388		
(SD)	(0.063809)	(0.064357)	(0.063339)	(0.037682)		
Median	0.13410	-0.09590	0.20910	-0.00230		
Min, Max	0.0854, 0.3244	-0.1938, 0.0651	0.1000, 0.2644	-0.0604, 0.0411		
Week 10						
n	10	10	6	6		
Mean	0.16116	-0.07098	0.20203	0.00097		
(SD)	(0.071891)	(0.066174)	(0.061656)	(0.029230)		
Median	0.13400	-0.08440	0.18945	0.00485		
Min, Max	0.1027, 0.3238	-0.1823, 0.0645	0.1400, 0.2930	-0.0347, 0.0325		
Week 12						
n	11	11	6	6		
Mean	0.16307	-0.05121	0.21010	0.00903		
(SD)	(0.075249)	(0.074402)	(0.067771)	(0.039032)		
Median	0.15110	-0.05970	0.22395	0.01340		
Min, Max	0.0958, 0.3537	-0.1701, 0.0944	0.1000, 0.2908	-0.0340, 0.0730		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.3

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	8	8	5	5		
Mean	0.15629	-0.07151	0.13666	-0.07862		
(SD)	(0.054971)	(0.062261)	(0.032782)	(0.056300)		
Median	0.14490	-0.08420	0.12450	-0.05820		
Min, Max	0.0893, 0.2536	-0.1577, 0.0025	0.0966, 0.1802	-0.1663, -0.0192		
Week 14						
n	11	11	6	6		
Mean	0.13914	-0.08459	0.11653	-0.08453		
(SD)	(0.043272)	(0.043987)	(0.048507)	(0.070722)		
Median	0.13700	-0.08690	0.11205	-0.09000		
Min, Max	0.0642, 0.2066	-0.1441, -0.0303	0.0636, 0.2053	-0.2053, -0.0100		
Week 15						
n	12	12	5	5		
Mean	0.14972	-0.07509	0.09264	-0.11888		
(SD)	(0.049446)	(0.055675)	(0.017471)	(0.066962)		
Median	0.14835	-0.08910	0.09000	-0.11150		
Min, Max	0.0880, 0.2517	-0.1588, 0.0019	0.0702, 0.1143	-0.2196, -0.0400		

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Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.3

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	13	13	6	6		
Mean	0.16075	-0.06190	0.08532	-0.11575		
(SD)	(0.048925)	(0.049927)	(0.019569)	(0.061325)		
Median	0.16470	-0.05920	0.08700	-0.10025		
Min, Max	0.0819, 0.2540	-0.1412, 0.0076	0.0516, 0.1064	-0.2233, -0.0400		
Week 18						
n	11	11	6	6		
Mean	0.16205	-0.06167	0.11167	-0.08940		
(SD)	(0.054055)	(0.057694)	(0.032273)	(0.066249)		
Median	0.14840	-0.07360	0.11650	-0.09405		
Min, Max	0.0960, 0.2892	-0.1533, 0.0299	0.0689, 0.1548	-0.1915, 0.0060		
Week 20						
n	10	10	5	5		
Mean	0.13413	-0.08408	0.10140	-0.07492		
(SD)	(0.035035)	(0.048676)	(0.016152)	(0.026275)		
Median	0.13525	-0.09575	0.09940	-0.07660		
Min, Max	0.0838, 0.1934	-0.1469, 0.0048	0.0800, 0.1228	-0.1115, -0.0494		

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Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

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ALXN2040-PNH-301

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Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	12	12	4	4		
Mean	0.15065	-0.07416	0.11230	-0.07560		
(SD)	(0.064251)	(0.062223)	(0.035426)	(0.018816)		
Median	0.14055	-0.09245	0.11290	-0.08205		
Min, Max	0.0708, 0.2709	-0.1496, 0.0233	0.0729, 0.1505	-0.0894, -0.0489		
Week 24						
n	12	12	4	4		
Mean	0.14799	-0.07160	0.13430	-0.05360		
(SD)	(0.052913)	(0.062224)	(0.032488)	(0.057097)		
Median	0.12635	-0.08380	0.13035	-0.06735		
Min, Max	0.0748, 0.2408	-0.1533, 0.0452	0.1016, 0.1749	-0.1058, 0.0261		
Week 28						
n	10	10	4	4	14	14
Mean	0.13236	-0.08635	0.11753	-0.07038	0.12812	-0.08179
(SD)	(0.042913)	(0.047627)	(0.015032)	(0.030838)	(0.037087)	(0.042965)
Median	0.12465	-0.10075	0.11095	-0.06395	0.12000	-0.08940
Min, Max	0.0843, 0.2376	-0.1496, -0.0006	0.1084, 0.1398	-0.1132, -0.0404	0.0843, 0.2376	-0.1496, -0.0006

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

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Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	11	11	4	4	15	15
Mean	0.15819	-0.06306	0.09963	-0.06535	0.14257	-0.06367
(SD)	(0.052723)	(0.044333)	(0.026384)	(0.017522)	(0.053417)	(0.038351)
Median	0.15320	-0.07680	0.09945	-0.07075	0.13070	-0.07280
Min, Max	0.0882, 0.2538	-0.1204, 0.0014	0.0689, 0.1307	-0.0799, -0.0400	0.0689, 0.2538	-0.1204, 0.0014
Week 36						
n	12	12	3	3	15	15
Mean	0.15311	-0.06837	0.11503	-0.06160	0.14549	-0.06701
(SD)	(0.072273)	(0.075081)	(0.036407)	(0.012541)	(0.067394)	(0.066779)
Median	0.14050	-0.06950	0.13080	-0.05850	0.13080	-0.06360
Min, Max	0.0750, 0.3384	-0.1888, 0.0860	0.0734, 0.1409	-0.0754, -0.0509	0.0734, 0.3384	-0.1888, 0.0860
Week 40						
n	11	11	3	3	14	14
Mean	0.16015	-0.06646	0.10287	-0.07377	0.14787	-0.06803
(SD)	(0.049214)	(0.035921)	(0.040001)	(0.017602)	(0.052001)	(0.032402)
Median	0.15580	-0.08200	0.12470	-0.07220	0.13510	-0.07710
Min, Max	0.0898, 0.2358	-0.1111, -0.0052	0.0567, 0.1272	-0.0921, -0.0570	0.0567, 0.2358	-0.1111, -0.0052

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Post-hoc Analysen Studie ALPHA

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≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	11	11	2	2	13	13
Mean	0.15287	-0.07374	0.12550	-0.06505	0.14866	-0.07240
(SD)	(0.062184)	(0.054238)	(0.002263)	(0.014779)	(0.057693)	(0.049803)
Median	0.13340	-0.06860	0.12550	-0.06505	0.13200	-0.06860
Min, Max	0.0868, 0.3057	-0.1593, 0.0464	0.1239, 0.1271	-0.0755, -0.0546	0.0868, 0.3057	-0.1593, 0.0464
Week 48						
n	8	8	2	2	10	10
Mean	0.14070	-0.08305	0.12585	-0.06470	0.13773	-0.07938
(SD)	(0.041153)	(0.059875)	(0.015627)	(0.003111)	(0.037196)	(0.053378)
Median	0.12365	-0.09085	0.12585	-0.06470	0.12365	-0.06915
Min, Max	0.0903, 0.2020	-0.1630, 0.0105	0.1148, 0.1369	-0.0669, -0.0625	0.0903, 0.2020	-0.1630, 0.0105
Week 52						
n	8	8	1	1	9	9
Mean	0.14231	-0.08144	0.06000	-0.07000	0.13317	-0.08017
(SD)	(0.040481)	(0.047670)	(NA)	(NA)	(0.046762)	(0.044754)
Median	0.14080	-0.09415	0.06000	-0.07000	0.13680	-0.09110
Min, Max	0.0764, 0.2012	-0.1482, -0.0212	0.0600, 0.0600	-0.0700, -0.0700	0.0600, 0.2012	-0.1482, -0.0212

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Post-hoc Analysen Studie ALPHA

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≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	8	8	2	2	10	10
Mean	0.16489	-0.06734	0.10225	-0.05360	0.15236	-0.06459
(SD)	(0.049960)	(0.038291)	(0.031466)	(0.005091)	(0.052430)	(0.034305)
Median	0.15765	-0.06325	0.10225	-0.05360	0.12980	-0.05740
Min, Max	0.0996, 0.2323	-0.1322, -0.0231	0.0800, 0.1245	-0.0572, -0.0500	0.0800, 0.2323	-0.1322, -0.0231
Week 60						
n	9	9	2	2	11	11
Mean	0.16042	-0.06860	0.11730	-0.03855	0.15258	-0.06314
(SD)	(0.050277)	(0.045945)	(0.010324)	(0.026234)	(0.048345)	(0.043650)
Median	0.16680	-0.07910	0.11730	-0.03855	0.12460	-0.05710
Min, Max	0.0974, 0.2375	-0.1366, -0.0025	0.1100, 0.1246	-0.0571, -0.0200	0.0974, 0.2375	-0.1366, -0.0025
Week 64						
n	6	6	2	2	8	8
Mean	0.13242	-0.09582	0.07965	-0.07620	0.11923	-0.09091
(SD)	(0.039367)	(0.052767)	(0.041931)	(0.005374)	(0.044213)	(0.045557)
Median	0.12580	-0.10940	0.07965	-0.07620	0.12225	-0.09075
Min, Max	0.0833, 0.2034	-0.1593, -0.0079	0.0500, 0.1093	-0.0800, -0.0724	0.0500, 0.2034	-0.1593, -0.0079

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.3

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	5	5	1	1	6	6
Mean	0.13532	-0.09290	0.05000	-0.08000	0.12110	-0.09075
(SD)	(0.028649)	(0.039844)	(NA)	(NA)	(0.043242)	(0.036025)
Median	0.13820	-0.09960	0.05000	-0.08000	0.12515	-0.08980
Min, Max	0.1010, 0.1683	-0.1468, -0.0430	0.0500, 0.0500	-0.0800, -0.0800	0.0500, 0.1683	-0.1468, -0.0430
Week 72						
n	4	4	0	0	4	4
Mean	0.17248	-0.05998			0.17248	-0.05998
(SD)	(0.075611)	(0.041002)			(0.075611)	(0.041002)
Median	0.13915	-0.07310			0.13915	-0.07310
Min, Max	0.1268, 0.2848	-0.0935, -0.0002			0.1268, 0.2848	-0.0935, -0.0002
Week 80						
n	3	3	0	0	3	3
Mean	0.10737	-0.10757			0.10737	-0.10757
(SD)	(0.021621)	(0.010688)			(0.021621)	(0.010688)
Median	0.11530	-0.10860			0.11530	-0.10860
Min, Max	0.0829, 0.1239	-0.1177, -0.0964			0.0829, 0.1239	-0.1177, -0.0964

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB
Run Date: 2024-04-26T15:53:42

Data cutoff date of 31Mar2023
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.3

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	3	3	0	0	3	3
Mean	0.16817	-0.06833			0.16817	-0.06833
(SD)	(0.051334)	(0.019276)			(0.051334)	(0.019276)
Median	0.17540	-0.06950			0.17540	-0.06950
Min, Max	0.1136, 0.2155	-0.0870, -0.0485			0.1136, 0.2155	-0.0870, -0.0485
Week 96						
n	1	1	0	0	1	1
Mean	0.18940	-0.09560			0.18940	-0.09560
(SD)	(NA)	(NA)			(NA)	(NA)
Median	0.18940	-0.09560			0.18940	-0.09560
Min, Max	0.1894, 0.1894	-0.0956, -0.0956			0.1894, 0.1894	-0.0956, -0.0956
Week 104						
n	1	1	0	0	1	1
Mean	0.17020	-0.11480			0.17020	-0.11480
(SD)	(NA)	(NA)			(NA)	(NA)
Median	0.17020	-0.11480			0.17020	-0.11480
Min, Max	0.1702, 0.1702	-0.1148, -0.1148			0.1702, 0.1702	-0.1148, -0.1148

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.4
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	25		12		37	
Mean	0.23134		0.25103		0.23772	
(SD)	(0.067304)		(0.112525)		(0.083523)	
Median	0.22370		0.22750		0.22370	
Min, Max	0.1094, 0.3889		0.1112, 0.4782		0.1094, 0.4782	
Week 1						
n	22	22	9	8		
Mean	0.16072	-0.07224	0.23172	0.01533		
(SD)	(0.053271)	(0.053322)	(0.077292)	(0.027130)		
Median	0.15815	-0.06920	0.22300	0.01620		
Min, Max	0.0553, 0.2400	-0.2067, 0.0017	0.1507, 0.3962	-0.0274, 0.0499		
Week 2						
n	23	23	11	10		
Mean	0.11923	-0.11528	0.24673	0.00095		
(SD)	(0.053150)	(0.079211)	(0.083718)	(0.031362)		
Median	0.10490	-0.09880	0.23750	-0.00700		
Min, Max	0.0388, 0.2299	-0.3258, 0.0089	0.1367, 0.3825	-0.0507, 0.0686		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Source: ADaM.ADSL, ADaM.ADLB
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.4
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	20	20	10	10		
Mean	0.12287	-0.10103	0.22341	-0.00906		
(SD)	(0.058992)	(0.059053)	(0.075263)	(0.045605)		
Median	0.10795	-0.11575	0.22090	-0.00465		
Min, Max	0.0534, 0.2479	-0.1955, 0.0148	0.1013, 0.3531	-0.1185, 0.0529		
Week 4						
n	23	23	10	10		
Mean	0.12352	-0.11073	0.22685	-0.02096		
(SD)	(0.063560)	(0.093946)	(0.088818)	(0.052606)		
Median	0.11140	-0.10940	0.22115	-0.01600		
Min, Max	0.0326, 0.2846	-0.3350, 0.1083	0.1071, 0.3644	-0.1138, 0.0579		
Week 6						
n	18	18	11	11		
Mean	0.12184	-0.09250	0.25642	0.00161		
(SD)	(0.050751)	(0.061496)	(0.097124)	(0.038649)		
Median	0.09980	-0.10400	0.24660	0.00990		
Min, Max	0.0649, 0.2465	-0.1870, 0.0255	0.1089, 0.4066	-0.0716, 0.0503		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.4
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	19	19	10	10		
Mean	0.12670	-0.09087	0.22444	-0.00803		
(SD)	(0.055499)	(0.060547)	(0.074636)	(0.044872)		
Median	0.11120	-0.09870	0.23110	-0.01785		
Min, Max	0.0712, 0.3244	-0.1938, 0.0651	0.1179, 0.3592	-0.0740, 0.0764		
Week 10						
n	16	16	10	10		
Mean	0.14880	-0.07502	0.22264	-0.00458		
(SD)	(0.067704)	(0.067244)	(0.057467)	(0.058814)		
Median	0.13330	-0.07140	0.22195	0.00205		
Min, Max	0.0534, 0.3238	-0.1823, 0.0645	0.1274, 0.2930	-0.1417, 0.0855		
Week 12						
n	17	17	10	10		
Mean	0.15032	-0.06835	0.25546	-0.00882		
(SD)	(0.069323)	(0.070359)	(0.097492)	(0.058635)		
Median	0.12540	-0.05970	0.26225	-0.01140		
Min, Max	0.0612, 0.3537	-0.2054, 0.0944	0.1311, 0.4441	-0.1279, 0.0801		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.4
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	14	14	11	10		
Mean	0.14329	-0.07477	0.18985	-0.05481		
(SD)	(0.054190)	(0.068672)	(0.057053)	(0.091792)		
Median	0.13895	-0.06255	0.18550	-0.03215		
Min, Max	0.0495, 0.2536	-0.2346, 0.0076	0.0911, 0.3126	-0.2200, 0.0743		
Week 14						
n	13	13	12	12		
Mean	0.14034	-0.07510	0.13433	-0.11669		
(SD)	(0.042768)	(0.047940)	(0.031459)	(0.117527)		
Median	0.14090	-0.05280	0.13160	-0.09695		
Min, Max	0.0804, 0.2066	-0.1501, -0.0180	0.0655, 0.1988	-0.3603, 0.0290		
Week 15						
n	15	15	11	10		
Mean	0.14573	-0.06739	0.13441	-0.11032		
(SD)	(0.053018)	(0.052574)	(0.038110)	(0.084350)		
Median	0.12620	-0.07520	0.12880	-0.11350		
Min, Max	0.0813, 0.2517	-0.1588, 0.0189	0.0827, 0.2208	-0.2673, 0.0114		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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Table GERMANY.2.6.4.4

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	18	18	12	11		
Mean	0.16171	-0.06930	0.14355	-0.11317		
(SD)	(0.058068)	(0.070601)	(0.061779)	(0.088257)		
Median	0.15225	-0.06055	0.12940	-0.08930		
Min, Max	0.0819, 0.2986	-0.2473, 0.0749	0.0644, 0.3029	-0.2719, 0.0193		
Week 18						
n	17	17	9	8		
Mean	0.17218	-0.04778	0.15473	-0.11058		
(SD)	(0.066796)	(0.061857)	(0.065028)	(0.084932)		
Median	0.17040	-0.03160	0.16380	-0.11955		
Min, Max	0.0761, 0.2993	-0.1533, 0.0756	0.0509, 0.2377	-0.2256, 0.0050		
Week 20						
n	16	16	8	7		
Mean	0.14456	-0.08427	0.17430	-0.12364		
(SD)	(0.063041)	(0.075503)	(0.091694)	(0.054718)		
Median	0.13525	-0.07755	0.14105	-0.13060		
Min, Max	0.0538, 0.2949	-0.2686, 0.0712	0.0547, 0.3278	-0.2192, -0.0487		

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Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.4
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	18	18	8	7		
Mean	0.16561	-0.06828	0.15828	-0.09406		
(SD)	(0.066976)	(0.076078)	(0.060117)	(0.094925)		
Median	0.14625	-0.08170	0.15625	-0.08720		
Min, Max	0.0727, 0.2932	-0.2434, 0.0695	0.0871, 0.2625	-0.2533, 0.0531		
Week 24						
n	18	18	6	6		
Mean	0.14269	-0.07086	0.18837	-0.12592		
(SD)	(0.060794)	(0.044952)	(0.058326)	(0.141185)		
Median	0.13250	-0.06165	0.17395	-0.10440		
Min, Max	0.0351, 0.2444	-0.1533, 0.0011	0.1285, 0.2996	-0.2964, 0.0902		
Week 28						
n	15	15	6	5	21	20
Mean	0.14991	-0.06069	0.14163	-0.11602	0.14754	-0.07452
(SD)	(0.048062)	(0.044054)	(0.035646)	(0.093010)	(0.044151)	(0.062093)
Median	0.13180	-0.06440	0.13950	-0.08510	0.13180	-0.07225
Min, Max	0.0932, 0.2376	-0.1496, 0.0050	0.0968, 0.1874	-0.2632, -0.0166	0.0932, 0.2376	-0.2632, 0.0050

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Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.4
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	14	14	6	5	20	19
Mean	0.15414	-0.04117	0.15990	-0.10580	0.15587	-0.05818
(SD)	(0.059021)	(0.038267)	(0.072557)	(0.128882)	(0.061450)	(0.074858)
Median	0.14450	-0.03465	0.14235	-0.14580	0.14450	-0.04190
Min, Max	0.0748, 0.2538	-0.1204, 0.0260	0.0977, 0.2940	-0.2627, 0.0846	0.0748, 0.2940	-0.2627, 0.0846
Week 36						
n	12	12	8	8	20	20
Mean	0.16088	-0.07230	0.16194	-0.11218	0.16131	-0.08825
(SD)	(0.071259)	(0.068638)	(0.062046)	(0.078049)	(0.066018)	(0.073304)
Median	0.15150	-0.08310	0.15840	-0.07310	0.15840	-0.07600
Min, Max	0.0701, 0.3384	-0.1888, 0.0860	0.0928, 0.2678	-0.2388, -0.0404	0.0701, 0.3384	-0.2388, 0.0860
Week 40						
n	12	12	4	4	16	16
Mean	0.15710	-0.04853	0.22710	-0.12650	0.17460	-0.06803
(SD)	(0.050205)	(0.027388)	(0.129484)	(0.057070)	(0.078623)	(0.049165)
Median	0.14275	-0.03830	0.19615	-0.11785	0.15610	-0.06295
Min, Max	0.0839, 0.2358	-0.0943, -0.0166	0.1109, 0.4052	-0.1973, -0.0730	0.0839, 0.4052	-0.1973, -0.0166

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Source: ADaM.ADSL, ADaM.ADLB
Run Date: 2024-04-26T15:53:45
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.4
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	13	13	8	8	21	21
Mean	0.15789	-0.06265	0.19625	-0.07786	0.17250	-0.06844
(SD)	(0.063387)	(0.051657)	(0.072528)	(0.102528)	(0.067943)	(0.073059)
Median	0.14350	-0.05810	0.20120	-0.07205	0.16540	-0.05980
Min, Max	0.0675, 0.3057	-0.1473, 0.0464	0.1018, 0.3040	-0.2264, 0.0721	0.0675, 0.3057	-0.2264, 0.0721
Week 48						
n	10	10	6	5	16	15
Mean	0.15577	-0.06882	0.16815	-0.17014	0.16041	-0.10259
(SD)	(0.045434)	(0.054007)	(0.071521)	(0.088778)	(0.054607)	(0.081063)
Median	0.15040	-0.07885	0.16215	-0.13510	0.15790	-0.09910
Min, Max	0.0903, 0.2136	-0.1630, 0.0105	0.0788, 0.2721	-0.3072, -0.0943	0.0788, 0.2721	-0.3072, 0.0105
Week 52						
n	7	7	5	4	12	11
Mean	0.15507	-0.05896	0.15190	-0.12013	0.15375	-0.08120
(SD)	(0.062178)	(0.059527)	(0.088525)	(0.028202)	(0.070435)	(0.057594)
Median	0.14720	-0.02120	0.11550	-0.12920	0.13375	-0.09720
Min, Max	0.0764, 0.2693	-0.1482, -0.0029	0.0938, 0.3085	-0.1428, -0.0793	0.0764, 0.3085	-0.1482, -0.0029

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.4
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	8	8	5	4	13	12
Mean	0.17278	-0.05859	0.18942	-0.15805	0.17918	-0.09174
(SD)	(0.076082)	(0.035573)	(0.056916)	(0.074967)	(0.067286)	(0.068821)
Median	0.19250	-0.06325	0.16680	-0.14850	0.17670	-0.08445
Min, Max	0.0439, 0.2662	-0.1010, -0.0079	0.1240, 0.2705	-0.2573, -0.0779	0.0439, 0.2705	-0.2573, -0.0079
Week 60						
n	7	7	5	5	12	12
Mean	0.17144	-0.06126	0.16974	-0.07876	0.17073	-0.06855
(SD)	(0.057347)	(0.035637)	(0.083052)	(0.059953)	(0.065596)	(0.045618)
Median	0.16680	-0.06450	0.13640	-0.07330	0.15920	-0.06890
Min, Max	0.0974, 0.2375	-0.1112, -0.0218	0.1079, 0.3121	-0.1504, 0.0032	0.0974, 0.3121	-0.1504, 0.0032
Week 64						
n	5	5	5	5	10	10
Mean	0.15974	-0.07054	0.16566	-0.12242	0.16270	-0.09648
(SD)	(0.094781)	(0.074026)	(0.078017)	(0.107678)	(0.081900)	(0.091303)
Median	0.12590	-0.06560	0.15360	-0.09110	0.13230	-0.07835
Min, Max	0.0833, 0.3251	-0.1593, 0.0354	0.0925, 0.2906	-0.2974, -0.0187	0.0833, 0.3251	-0.2974, 0.0354

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.4
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	5	5	4	4	9	9
Mean	0.15876	-0.09308	0.15655	-0.10075	0.15778	-0.09649
(SD)	(0.073340)	(0.063625)	(0.100120)	(0.055126)	(0.080311)	(0.056391)
Median	0.13820	-0.09960	0.11950	-0.08940	0.13820	-0.09810
Min, Max	0.1010, 0.2867	-0.1565, -0.0030	0.0861, 0.3011	-0.1771, -0.0471	0.0861, 0.3011	-0.1771, -0.0030
Week 72						
n	4	4	5	5	9	9
Mean	0.18005	-0.06233	0.21530	-0.07278	0.19963	-0.06813
(SD)	(0.070845)	(0.064491)	(0.127658)	(0.054784)	(0.101860)	(0.055594)
Median	0.15345	-0.05040	0.13830	-0.07360	0.15100	-0.07210
Min, Max	0.1285, 0.2848	-0.1483, -0.0002	0.1032, 0.3596	-0.1290, 0.0150	0.1032, 0.3596	-0.1483, 0.0150
Week 80						
n	3	3	4	4	7	7
Mean	0.14433	-0.08383	0.12240	-0.19443	0.13180	-0.14703
(SD)	(0.053210)	(0.065172)	(0.050954)	(0.124578)	(0.048779)	(0.112561)
Median	0.17420	-0.11770	0.13470	-0.20425	0.14100	-0.11770
Min, Max	0.0829, 0.1759	-0.1251, -0.0087	0.0505, 0.1697	-0.3085, -0.0607	0.0505, 0.1759	-0.3085, -0.0087

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.4
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	3	3	2	2	5	5
Mean	0.17367	-0.04973	0.23955	-0.09940	0.20002	-0.06960
(SD)	(0.053341)	(0.050161)	(0.058478)	(0.074812)	(0.059831)	(0.058287)
Median	0.19190	-0.06950	0.23955	-0.09940	0.19820	-0.06950
Min, Max	0.1136, 0.2155	-0.0870, 0.0073	0.1982, 0.2809	-0.1523, -0.0465	0.1136, 0.2809	-0.1523, 0.0073
Week 96						
n	2	2	3	3	5	5
Mean	0.16840	-0.06640	0.10397	-0.15907	0.12974	-0.12200
(SD)	(0.029698)	(0.041295)	(0.032162)	(0.147447)	(0.044533)	(0.117783)
Median	0.16840	-0.06640	0.10870	-0.11120	0.13350	-0.09560
Min, Max	0.1474, 0.1894	-0.0956, -0.0372	0.0697, 0.1335	-0.3245, -0.0415	0.0697, 0.1894	-0.3245, -0.0372
Week 104						
n	1	1	2	2	3	3
Mean	0.17020	-0.11480	0.13280	-0.05615	0.14527	-0.07570
(SD)	(NA)	(NA)	(0.080327)	(0.001485)	(0.060766)	(0.033878)
Median	0.17020	-0.11480	0.13280	-0.05615	0.17020	-0.05720
Min, Max	0.1702, 0.1702	-0.1148, -0.1148	0.0760, 0.1896	-0.0572, -0.0551	0.0760, 0.1896	-0.1148, -0.0551

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.4
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		13		45	
Mean	0.26033		0.18908		0.23975	
(SD)	(0.114781)		(0.120120)		(0.119516)	
Median	0.21580		0.17380		0.19960	
Min, Max	0.1200, 0.5295		0.0394, 0.5419		0.0394, 0.5419	
Week 1						
n	25	25	12	12		
Mean	0.17370	-0.08266	0.18991	-0.00243		
(SD)	(0.066207)	(0.072287)	(0.105140)	(0.048009)		
Median	0.16910	-0.08250	0.18585	0.00270		
Min, Max	0.0789, 0.3200	-0.2457, 0.0183	0.0410, 0.4444	-0.0975, 0.0929		
Week 2						
n	30	30	11	11		
Mean	0.12729	-0.13621	0.19506	0.00539		
(SD)	(0.058642)	(0.113003)	(0.111088)	(0.034425)		
Median	0.12250	-0.10005	0.16930	0.01000		
Min, Max	0.0419, 0.3474	-0.4371, -0.0213	0.0402, 0.4748	-0.0671, 0.0619		

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Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
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Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	26	26	10	10		
Mean	0.10970	-0.14117	0.19668	0.00104		
(SD)	(0.055275)	(0.110243)	(0.127479)	(0.028102)		
Median	0.10165	-0.09965	0.17735	-0.00225		
Min, Max	0.0400, 0.2506	-0.4571, -0.0104	0.0309, 0.5193	-0.0478, 0.0443		
Week 4						
n	27	27	11	11		
Mean	0.12207	-0.12391	0.19080	0.00113		
(SD)	(0.048377)	(0.096905)	(0.137395)	(0.038772)		
Median	0.11050	-0.11950	0.14710	0.01660		
Min, Max	0.0529, 0.2300	-0.3445, 0.0100	0.0388, 0.5743	-0.0993, 0.0411		
Week 6						
n	29	29	11	11		
Mean	0.13461	-0.11103	0.18845	-0.00123		
(SD)	(0.052480)	(0.083286)	(0.129796)	(0.029196)		
Median	0.12150	-0.08290	0.17810	-0.00410		
Min, Max	0.0554, 0.3100	-0.3016, -0.0146	0.0343, 0.5254	-0.0464, 0.0611		

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Post-hoc Analysen Studie ALPHA

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Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	25	25	12	12		
Mean	0.14558	-0.09691	0.19723	0.00488		
(SD)	(0.044751)	(0.085548)	(0.104367)	(0.044374)		
Median	0.15150	-0.07590	0.18535	0.00225		
Min, Max	0.0682, 0.2300	-0.2995, 0.0106	0.0424, 0.4560	-0.0859, 0.0827		
Week 10						
n	21	21	10	10		
Mean	0.15869	-0.11229	0.22182	0.02167		
(SD)	(0.044580)	(0.081100)	(0.135032)	(0.031227)		
Median	0.14900	-0.09070	0.17070	0.02195		
Min, Max	0.0785, 0.2299	-0.2859, 0.0068	0.1180, 0.5817	-0.0347, 0.0826		
Week 12						
n	28	28	12	12		
Mean	0.15555	-0.10648	0.20436	0.01202		
(SD)	(0.050458)	(0.105375)	(0.125433)	(0.041266)		
Median	0.15300	-0.08645	0.18765	0.00615		
Min, Max	0.0800, 0.2900	-0.3279, 0.0311	0.0383, 0.5375	-0.0597, 0.0948		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	24	24	9	9		
Mean	0.15629	-0.09805	0.13102	-0.02109		
(SD)	(0.053712)	(0.104270)	(0.047492)	(0.052733)		
Median	0.15135	-0.07655	0.14270	-0.03110		
Min, Max	0.0796, 0.3100	-0.3539, 0.0460	0.0291, 0.1883	-0.0972, 0.0841		
Week 14						
n	27	27	10	10		
Mean	0.15270	-0.10843	0.10926	-0.04064		
(SD)	(0.047754)	(0.101226)	(0.049238)	(0.050893)		
Median	0.15260	-0.08690	0.10545	-0.04195		
Min, Max	0.0642, 0.2720	-0.3537, 0.0100	0.0241, 0.2053	-0.0955, 0.0501		
Week 15						
n	24	24	8	8		
Mean	0.14965	-0.09312	0.09556	-0.05821		
(SD)	(0.043643)	(0.105815)	(0.038261)	(0.062934)		
Median	0.14355	-0.08925	0.09160	-0.07125		
Min, Max	0.0736, 0.2437	-0.3686, 0.0437	0.0328, 0.1641	-0.1382, 0.0599		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.4
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	24	24	11	11		
Mean	0.14901	-0.09289	0.09535	-0.06521		
(SD)	(0.037840)	(0.094487)	(0.037375)	(0.059389)		
Median	0.15270	-0.05745	0.09660	-0.08340		
Min, Max	0.0832, 0.2236	-0.3234, 0.0240	0.0315, 0.1681	-0.1377, 0.0639		
Week 18						
n	22	22	9	9		
Mean	0.16671	-0.08125	0.10728	-0.04594		
(SD)	(0.057349)	(0.081966)	(0.035393)	(0.052975)		
Median	0.15305	-0.08020	0.11710	-0.05540		
Min, Max	0.0811, 0.3164	-0.2201, 0.0800	0.0461, 0.1548	-0.1128, 0.0396		
Week 20						
n	25	25	9	9		
Mean	0.16070	-0.09550	0.10336	-0.04987		
(SD)	(0.053058)	(0.083136)	(0.032032)	(0.046647)		
Median	0.15570	-0.07340	0.11020	-0.05440		
Min, Max	0.0833, 0.2900	-0.3087, 0.0419	0.0362, 0.1500	-0.1115, 0.0458		

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:53:45

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.4
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	22	22	9	9		
Mean	0.15320	-0.09042	0.11668	-0.05179		
(SD)	(0.042003)	(0.101605)	(0.043515)	(0.039559)		
Median	0.15990	-0.07115	0.12040	-0.06030		
Min, Max	0.0708, 0.2451	-0.3880, 0.0455	0.0421, 0.1916	-0.0894, 0.0229		
Week 24						
n	24	24	8	8		
Mean	0.15217	-0.09298	0.14984	-0.03476		
(SD)	(0.061147)	(0.095498)	(0.038340)	(0.047958)		
Median	0.13745	-0.06685	0.14445	-0.03480		
Min, Max	0.0748, 0.3700	-0.3735, 0.0452	0.1016, 0.2273	-0.1058, 0.0346		
Week 28						
n	24	24	8	8	32	32
Mean	0.15123	-0.09528	0.13184	-0.05276	0.14638	-0.08465
(SD)	(0.075442)	(0.071222)	(0.021120)	(0.049271)	(0.066304)	(0.068276)
Median	0.14280	-0.09225	0.13150	-0.05510	0.14140	-0.07575
Min, Max	0.0533, 0.4700	-0.2643, -0.0006	0.1084, 0.1621	-0.1132, 0.0455	0.0533, 0.4700	-0.2643, 0.0455

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.4
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	26	26	8	8	34	34
Mean	0.16334	-0.09902	0.12376	-0.04938	0.15403	-0.08734
(SD)	(0.056267)	(0.101585)	(0.036157)	(0.052815)	(0.054462)	(0.094161)
Median	0.15870	-0.08250	0.13030	-0.05435	0.14765	-0.07075
Min, Max	0.0526, 0.2757	-0.3290, 0.0531	0.0689, 0.1900	-0.1373, 0.0289	0.0526, 0.2757	-0.3290, 0.0531
Week 36						
n	21	21	8	8	29	29
Mean	0.15429	-0.10033	0.12956	-0.04233	0.14747	-0.08433
(SD)	(0.044866)	(0.089763)	(0.031781)	(0.039357)	(0.042624)	(0.082697)
Median	0.15050	-0.07380	0.13520	-0.04765	0.14380	-0.05850
Min, Max	0.0750, 0.2563	-0.3076, 0.0091	0.0734, 0.1723	-0.0949, 0.0354	0.0734, 0.2563	-0.3076, 0.0354
Week 40						
n	23	23	7	7	30	30
Mean	0.16445	-0.09375	0.12866	-0.05064	0.15610	-0.08369
(SD)	(0.047092)	(0.093635)	(0.034168)	(0.047704)	(0.046487)	(0.086406)
Median	0.17010	-0.08200	0.13940	-0.05700	0.15055	-0.07490
Min, Max	0.0858, 0.2831	-0.2646, 0.0538	0.0567, 0.1624	-0.1048, 0.0382	0.0567, 0.2831	-0.2646, 0.0538

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.4
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	21	21	6	6	27	27
Mean	0.16269	-0.09883	0.14443	-0.03995	0.15863	-0.08574
(SD)	(0.058717)	(0.084129)	(0.025407)	(0.043635)	(0.053254)	(0.080205)
Median	0.15460	-0.06660	0.13115	-0.05035	0.15150	-0.06440
Min, Max	0.0845, 0.3216	-0.3035, -0.0146	0.1239, 0.1818	-0.0854, 0.0304	0.0845, 0.3216	-0.3035, 0.0304
Week 48						
n	19	19	6	6	25	25
Mean	0.16863	-0.08598	0.13118	-0.05320	0.15964	-0.07812
(SD)	(0.053640)	(0.071856)	(0.015650)	(0.044628)	(0.049754)	(0.067019)
Median	0.16200	-0.06190	0.12845	-0.05895	0.13690	-0.06190
Min, Max	0.1072, 0.2753	-0.2917, 0.0006	0.1148, 0.1586	-0.1086, 0.0281	0.1072, 0.2753	-0.2917, 0.0281
Week 52						
n	16	16	5	5	21	21
Mean	0.15241	-0.08373	0.12562	-0.04542	0.14603	-0.07460
(SD)	(0.035668)	(0.066486)	(0.050775)	(0.037964)	(0.040081)	(0.062314)
Median	0.14650	-0.07865	0.11700	-0.07000	0.14480	-0.07000
Min, Max	0.0871, 0.2081	-0.2386, -0.0200	0.0600, 0.1938	-0.0734, 0.0128	0.0600, 0.2081	-0.2386, 0.0128

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.4
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	21	21	5	5	26	26
Mean	0.15196	-0.10208	0.12174	-0.06480	0.14615	-0.09491
(SD)	(0.049954)	(0.088166)	(0.024841)	(0.033323)	(0.047355)	(0.081368)
Median	0.13110	-0.09370	0.12450	-0.05040	0.12980	-0.08145
Min, Max	0.0736, 0.2581	-0.3276, -0.0100	0.0800, 0.1435	-0.1237, -0.0427	0.0736, 0.2581	-0.3276, -0.0100
Week 60						
n	15	15	6	6	21	21
Mean	0.16462	-0.10674	0.13802	-0.03480	0.15702	-0.08619
(SD)	(0.053439)	(0.089313)	(0.024092)	(0.052567)	(0.047914)	(0.085928)
Median	0.14830	-0.09630	0.13870	-0.04215	0.14830	-0.05710
Min, Max	0.0906, 0.2700	-0.2899, -0.0025	0.1100, 0.1658	-0.1014, 0.0550	0.0906, 0.2700	-0.2899, 0.0550
Week 64						
n	14	14	6	6	20	20
Mean	0.15265	-0.10750	0.12622	-0.04660	0.14472	-0.08923
(SD)	(0.076559)	(0.085932)	(0.044256)	(0.043177)	(0.068412)	(0.079768)
Median	0.14040	-0.10325	0.13125	-0.05320	0.13860	-0.08110
Min, Max	0.0500, 0.3500	-0.2752, -0.0079	0.0500, 0.1728	-0.0944, 0.0185	0.0500, 0.3500	-0.2752, 0.0185

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.4
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	10	10	5	5	15	15
Mean	0.14195	-0.09846	0.12764	-0.04340	0.13718	-0.08011
(SD)	(0.058960)	(0.057310)	(0.053365)	(0.040780)	(0.055652)	(0.057518)
Median	0.13980	-0.08755	0.12480	-0.05780	0.13330	-0.07140
Min, Max	0.0637, 0.2565	-0.2357, -0.0430	0.0500, 0.1958	-0.0800, 0.0206	0.0500, 0.2565	-0.2357, 0.0206
Week 72						
n	10	10	3	3	13	13
Mean	0.17636	-0.10410	0.15090	-0.00177	0.17048	-0.08048
(SD)	(0.094400)	(0.080392)	(0.021100)	(0.043311)	(0.082960)	(0.084698)
Median	0.13990	-0.07620	0.14800	-0.00670	0.14800	-0.07000
Min, Max	0.0932, 0.3833	-0.2697, -0.0287	0.1314, 0.1733	-0.0424, 0.0438	0.0932, 0.3833	-0.2697, 0.0438
Week 80						
n	8	8	3	3	11	11
Mean	0.17746	-0.09336	0.12787	-0.02480	0.16394	-0.07466
(SD)	(0.085846)	(0.085872)	(0.012126)	(0.054211)	(0.075662)	(0.082311)
Median	0.13395	-0.07815	0.12210	-0.05170	0.13330	-0.05990
Min, Max	0.1027, 0.3500	-0.2474, 0.0200	0.1197, 0.1418	-0.0603, 0.0376	0.1027, 0.3500	-0.2474, 0.0376

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.4
Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L)
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	3	3	1	1	4	4
Mean	0.13940	-0.17563	0.19700	0.02320	0.15380	-0.12593
(SD)	(0.052790)	(0.164336)	(NA)	(NA)	(0.051839)	(0.166996)
Median	0.16400	-0.11720	0.19700	0.02320	0.16970	-0.08285
Min, Max	0.0788, 0.1754	-0.3612, -0.0485	0.1970, 0.1970	0.0232, 0.0232	0.0788, 0.1970	-0.3612, 0.0232
Week 96						
n	2	2	1	1	3	3
Mean	0.17140	-0.06745	0.14320	-0.03060	0.16200	-0.05517
(SD)	(0.000849)	(0.059043)	(NA)	(NA)	(0.016292)	(0.046858)
Median	0.17140	-0.06745	0.14320	-0.03060	0.17080	-0.03060
Min, Max	0.1708, 0.1720	-0.1092, -0.0257	0.1432, 0.1432	-0.0306, -0.0306	0.1432, 0.1720	-0.1092, -0.0257

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.5

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		9		33	
Mean	0.22750		0.19386		0.21833	
(SD)	(0.082897)		(0.113565)		(0.091624)	
Median	0.20595		0.17310		0.20060	
Min, Max	0.1094, 0.4400		0.0394, 0.4332		0.0394, 0.4400	
Week 1						
n	20	20	8	7		
Mean	0.16934	-0.06553	0.18335	-0.00450		
(SD)	(0.051063)	(0.066972)	(0.113174)	(0.030160)		
Median	0.16310	-0.03000	0.17150	-0.00210		
Min, Max	0.0895, 0.2864	-0.2100, 0.0183	0.0410, 0.3962	-0.0473, 0.0499		
Week 2						
n	22	22	9	8		
Mean	0.13402	-0.09619	0.20504	-0.00871		
(SD)	(0.068286)	(0.092590)	(0.118203)	(0.020813)		
Median	0.12175	-0.06995	0.16630	-0.00700		
Min, Max	0.0418, 0.3474	-0.3609, 0.0089	0.0402, 0.3825	-0.0507, 0.0161		

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.5

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	18	18	7	7		
Mean	0.13187	-0.10033	0.18041	-0.02034		
(SD)	(0.062183)	(0.096108)	(0.094812)	(0.045396)		
Median	0.13375	-0.07655	0.16140	-0.00850		
Min, Max	0.0400, 0.2506	-0.4000, 0.0148	0.0309, 0.3147	-0.1185, 0.0231		
Week 4						
n	21	21	8	8		
Mean	0.12457	-0.08699	0.18740	-0.00451		
(SD)	(0.049893)	(0.080285)	(0.102983)	(0.043665)		
Median	0.11140	-0.05790	0.16000	-0.00030		
Min, Max	0.0326, 0.2011	-0.3350, -0.0067	0.0388, 0.3359	-0.0973, 0.0579		
Week 6						
n	20	20	8	8		
Mean	0.13132	-0.07768	0.18208	-0.00984		
(SD)	(0.050970)	(0.077892)	(0.116994)	(0.031870)		
Median	0.12660	-0.04945	0.15105	-0.00460		
Min, Max	0.0646, 0.2465	-0.3016, 0.0255	0.0343, 0.3763	-0.0569, 0.0273		

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	18	18	8	8		
Mean	0.13367	-0.06067	0.18258	-0.00934		
(SD)	(0.040378)	(0.056207)	(0.106347)	(0.033067)		
Median	0.12915	-0.06185	0.16030	-0.00065		
Min, Max	0.0839, 0.2109	-0.1938, 0.0106	0.0424, 0.3592	-0.0740, 0.0381		
Week 10						
n	14	14	7	7		
Mean	0.15836	-0.06845	0.19356	-0.01264		
(SD)	(0.042100)	(0.056693)	(0.068657)	(0.059303)		
Median	0.14875	-0.05935	0.15510	0.00990		
Min, Max	0.1027, 0.2299	-0.1905, 0.0068	0.1180, 0.2915	-0.1417, 0.0378		
Week 12						
n	21	21	9	9		
Mean	0.15315	-0.06589	0.19294	-0.00091		
(SD)	(0.047446)	(0.077009)	(0.103404)	(0.060790)		
Median	0.15110	-0.04280	0.16720	-0.00110		
Min, Max	0.0612, 0.2630	-0.3000, 0.0311	0.0383, 0.3346	-0.1279, 0.0801		

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	15	15	9	8		
Mean	0.16217	-0.05344	0.18474	-0.03308		
(SD)	(0.047946)	(0.080700)	(0.081188)	(0.090271)		
Median	0.15270	-0.03320	0.18830	-0.01625		
Min, Max	0.0893, 0.2456	-0.2800, 0.0460	0.0291, 0.3126	-0.2200, 0.0841		
Week 14						
n	18	18	9	9		
Mean	0.14559	-0.07831	0.12431	-0.06954		
(SD)	(0.044499)	(0.078242)	(0.051682)	(0.099038)		
Median	0.15300	-0.03680	0.13220	-0.02630		
Min, Max	0.0642, 0.2158	-0.2590, -0.0012	0.0241, 0.1988	-0.2852, 0.0501		
Week 15						
n	16	16	9	8		
Mean	0.15182	-0.06156	0.13636	-0.06594		
(SD)	(0.045798)	(0.081934)	(0.053256)	(0.102501)		
Median	0.15100	-0.02690	0.14670	-0.04050		
Min, Max	0.0880, 0.2399	-0.2845, 0.0189	0.0328, 0.2208	-0.2673, 0.0599		

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	17	17	9	8		
Mean	0.15809	-0.05966	0.12090	-0.07648		
(SD)	(0.038186)	(0.070027)	(0.058091)	(0.101572)		
Median	0.15540	-0.03610	0.12830	-0.05245		
Min, Max	0.0819, 0.2236	-0.2463, 0.0240	0.0315, 0.1970	-0.2719, 0.0639		
Week 18						
n	17	17	9	8		
Mean	0.16276	-0.04787	0.15198	-0.05519		
(SD)	(0.043713)	(0.062998)	(0.068108)	(0.092548)		
Median	0.17110	-0.02290	0.15480	-0.02250		
Min, Max	0.0865, 0.2428	-0.2138, 0.0432	0.0461, 0.2377	-0.2256, 0.0396		
Week 20						
n	16	16	9	8		
Mean	0.15375	-0.07006	0.13910	-0.07009		
(SD)	(0.052364)	(0.076095)	(0.072136)	(0.081360)		
Median	0.14920	-0.05210	0.12440	-0.04970		
Min, Max	0.0838, 0.2415	-0.2686, 0.0419	0.0362, 0.2773	-0.2192, 0.0458		

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	17	17	9	8		
Mean	0.15441	-0.06095	0.14260	-0.06776		
(SD)	(0.050796)	(0.077866)	(0.070142)	(0.099214)		
Median	0.13760	-0.03490	0.12710	-0.06705		
Min, Max	0.0835, 0.2451	-0.2762, 0.0455	0.0421, 0.2625	-0.2533, 0.0531		
Week 24						
n	17	17	6	6		
Mean	0.14217	-0.07028	0.18273	-0.05097		
(SD)	(0.052852)	(0.074279)	(0.061483)	(0.130124)		
Median	0.13330	-0.05880	0.17050	-0.02365		
Min, Max	0.0351, 0.2173	-0.2724, 0.0452	0.1285, 0.2996	-0.2671, 0.0902		
Week 28						
n	17	17	6	5	23	22
Mean	0.14088	-0.07258	0.14532	-0.08390	0.14203	-0.07515
(SD)	(0.037670)	(0.064905)	(0.035553)	(0.112743)	(0.036377)	(0.075195)
Median	0.14080	-0.06440	0.15465	-0.07630	0.14200	-0.06630
Min, Max	0.0843, 0.2260	-0.2564, 0.0050	0.0968, 0.1874	-0.2632, 0.0455	0.0843, 0.2260	-0.2632, 0.0455

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	18	18	8	7	26	25
Mean	0.16559	-0.05778	0.14236	-0.06774	0.15844	-0.06057
(SD)	(0.052072)	(0.084299)	(0.071215)	(0.113907)	(0.058166)	(0.091095)
Median	0.15760	-0.03395	0.12405	-0.05810	0.15185	-0.03470
Min, Max	0.0748, 0.2757	-0.3003, 0.0531	0.0689, 0.2940	-0.2627, 0.0846	0.0689, 0.2940	-0.3003, 0.0846
Week 36						
n	13	13	6	6	19	19
Mean	0.14166	-0.06251	0.14918	-0.07847	0.14404	-0.06755
(SD)	(0.044702)	(0.049962)	(0.054410)	(0.089254)	(0.046556)	(0.062730)
Median	0.13480	-0.05030	0.15385	-0.07250	0.13960	-0.05260
Min, Max	0.0750, 0.2563	-0.1888, 0.0091	0.0734, 0.2161	-0.2388, 0.0354	0.0734, 0.2563	-0.2388, 0.0354
Week 40						
n	15	15	4	4	19	19
Mean	0.15177	-0.07723	0.14785	-0.08488	0.15095	-0.07884
(SD)	(0.041268)	(0.079420)	(0.073403)	(0.096351)	(0.047173)	(0.080395)
Median	0.15580	-0.05490	0.14940	-0.09020	0.15580	-0.07100
Min, Max	0.0839, 0.2263	-0.2646, -0.0052	0.0567, 0.2359	-0.1973, 0.0382	0.0567, 0.2359	-0.2646, 0.0382

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> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	13	13	5	5	18	18
Mean	0.15241	-0.06375	0.19708	-0.05360	0.16482	-0.06093
(SD)	(0.051456)	(0.060161)	(0.054953)	(0.115629)	(0.054803)	(0.075648)
Median	0.13840	-0.04870	0.19630	-0.06560	0.15225	-0.05250
Min, Max	0.0675, 0.2713	-0.2311, -0.0066	0.1346, 0.2815	-0.2264, 0.0721	0.0675, 0.2815	-0.2311, 0.0721
Week 48						
n	12	12	5	4	17	16
Mean	0.16729	-0.05859	0.16338	-0.10260	0.16614	-0.06959
(SD)	(0.058025)	(0.057919)	(0.081423)	(0.099247)	(0.063052)	(0.069407)
Median	0.15040	-0.03675	0.13230	-0.11470	0.13830	-0.06110
Min, Max	0.0903, 0.2753	-0.1630, 0.0105	0.0788, 0.2721	-0.2091, 0.0281	0.0788, 0.2753	-0.2091, 0.0281
Week 52						
n	11	11	6	5	17	16
Mean	0.14421	-0.08144	0.13683	-0.07898	0.14161	-0.08067
(SD)	(0.045286)	(0.064727)	(0.087548)	(0.058286)	(0.060747)	(0.060831)
Median	0.14720	-0.09290	0.11400	-0.07930	0.13070	-0.08610
Min, Max	0.0764, 0.2081	-0.2135, -0.0029	0.0600, 0.3085	-0.1337, 0.0128	0.0600, 0.3085	-0.2135, 0.0128

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> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	9	9	4	3	13	12
Mean	0.16303	-0.05437	0.17055	-0.09687	0.16535	-0.06499
(SD)	(0.058115)	(0.040231)	(0.077964)	(0.058695)	(0.061516)	(0.046615)
Median	0.17670	-0.04290	0.16585	-0.07790	0.16680	-0.05380
Min, Max	0.0891, 0.2581	-0.1064, -0.0079	0.0800, 0.2705	-0.1627, -0.0500	0.0800, 0.2705	-0.1627, -0.0079
Week 60						
n	9	9	5	5	14	14
Mean	0.16234	-0.05094	0.17472	-0.04232	0.16676	-0.04786
(SD)	(0.050886)	(0.035178)	(0.080948)	(0.065637)	(0.060395)	(0.045886)
Median	0.15160	-0.04820	0.15920	-0.05220	0.15540	-0.05020
Min, Max	0.0906, 0.2477	-0.1041, -0.0025	0.1100, 0.3121	-0.1211, 0.0550	0.0906, 0.3121	-0.1211, 0.0550
Week 64						
n	8	8	5	5	13	13
Mean	0.13218	-0.07015	0.14554	-0.07150	0.13732	-0.07067
(SD)	(0.040210)	(0.055213)	(0.089392)	(0.058516)	(0.060437)	(0.054038)
Median	0.13230	-0.05575	0.12270	-0.08000	0.12590	-0.06560
Min, Max	0.0783, 0.2034	-0.1593, -0.0079	0.0500, 0.2906	-0.1426, 0.0185	0.0500, 0.2906	-0.1593, 0.0185

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.5

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	7	7	4	4	11	11
Mean	0.13527	-0.08954	0.10345	-0.05955	0.12370	-0.07864
(SD)	(0.063102)	(0.041774)	(0.042019)	(0.054085)	(0.056361)	(0.046407)
Median	0.12510	-0.09960	0.10860	-0.08035	0.12480	-0.08070
Min, Max	0.0637, 0.2565	-0.1468, -0.0430	0.0500, 0.1466	-0.0981, 0.0206	0.0500, 0.2565	-0.1468, 0.0206
Week 72						
n	6	6	4	4	10	10
Mean	0.20053	-0.04970	0.18728	-0.05153	0.19523	-0.05043
(SD)	(0.110506)	(0.035326)	(0.116484)	(0.065633)	(0.106555)	(0.046153)
Median	0.15670	-0.04785	0.14315	-0.07175	0.15195	-0.06330
Min, Max	0.0932, 0.3833	-0.1015, -0.0002	0.1032, 0.3596	-0.1064, 0.0438	0.0932, 0.3833	-0.1064, 0.0438
Week 80						
n	2	2	3	3	5	5
Mean	0.12940	-0.06320	0.13707	-0.12363	0.13400	-0.09946
(SD)	(0.065761)	(0.077075)	(0.007516)	(0.165022)	(0.033571)	(0.127267)
Median	0.12940	-0.06320	0.14100	-0.11630	0.14100	-0.11630
Min, Max	0.0829, 0.1759	-0.1177, -0.0087	0.1284, 0.1418	-0.2922, 0.0376	0.0829, 0.1759	-0.2922, 0.0376

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.5

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> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	4	4	2	2	6	6
Mean	0.14995	-0.12760	0.23955	-0.09940	0.17982	-0.11820
(SD)	(0.064395)	(0.161029)	(0.058478)	(0.074812)	(0.072889)	(0.129960)
Median	0.15275	-0.07825	0.23955	-0.09940	0.19505	-0.07825
Min, Max	0.0788, 0.2155	-0.3612, 0.0073	0.1982, 0.2809	-0.1523, -0.0465	0.0788, 0.2809	-0.3612, 0.0073
Week 96						
n	3	3	2	2	5	5
Mean	0.16920	-0.05283	0.12110	-0.21785	0.14996	-0.11884
(SD)	(0.021046)	(0.037481)	(0.017536)	(0.150826)	(0.031503)	(0.120659)
Median	0.17080	-0.03720	0.12110	-0.21785	0.14740	-0.09560
Min, Max	0.1474, 0.1894	-0.0956, -0.0257	0.1087, 0.1335	-0.3245, -0.1112	0.1087, 0.1894	-0.3245, -0.0257
Week 104						
n	1	1	1	1	2	2
Mean	0.17020	-0.11480	0.18960	-0.05510	0.17990	-0.08495
(SD)	(NA)	(NA)	(NA)	(NA)	(0.013718)	(0.042214)
Median	0.17020	-0.11480	0.18960	-0.05510	0.17990	-0.08495
Min, Max	0.1702, 0.1702	-0.1148, -0.1148	0.1896, 0.1896	-0.0551, -0.0551	0.1702, 0.1896	-0.1148, -0.0551

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.5

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		16		49	
Mean	0.26225		0.23286		0.25265	
(SD)	(0.105194)		(0.122319)		(0.110665)	
Median	0.23670		0.19055		0.22170	
Min, Max	0.1357, 0.5295		0.1112, 0.5419		0.1112, 0.5419	
Week 1						
n	27	27	13	13		
Mean	0.16636	-0.08686	0.22289	0.00961		
(SD)	(0.067117)	(0.060746)	(0.082149)	(0.046239)		
Median	0.15710	-0.08570	0.21680	0.01650		
Min, Max	0.0553, 0.3200	-0.2457, 0.0100	0.1400, 0.4444	-0.0975, 0.0929		
Week 2						
n	31	31	13	13		
Mean	0.11654	-0.14908	0.23187	0.01065		
(SD)	(0.045043)	(0.099670)	(0.087831)	(0.036420)		
Median	0.11100	-0.12870	0.20680	0.01720		
Min, Max	0.0388, 0.2100	-0.4371, -0.0300	0.1367, 0.4748	-0.0671, 0.0686		

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Post-hoc Analysen Studie ALPHA

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ALXN2040-PNH-301

Table GERMANY.2.6.4.5

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	28	28	13	13		
Mean	0.10485	-0.13875	0.22600	0.00478		
(SD)	(0.051184)	(0.089259)	(0.107012)	(0.030411)		
Median	0.09025	-0.11670	0.20830	0.00710		
Min, Max	0.0480, 0.2479	-0.4571, -0.0247	0.1013, 0.5193	-0.0478, 0.0529		
Week 4						
n	29	29	13	13		
Mean	0.12141	-0.14019	0.22062	-0.01239		
(SD)	(0.059714)	(0.099479)	(0.124791)	(0.049024)		
Median	0.11050	-0.15020	0.20250	0.01450		
Min, Max	0.0525, 0.2846	-0.3445, 0.1083	0.1071, 0.5743	-0.1138, 0.0411		
Week 6						
n	27	27	14	14		
Mean	0.12854	-0.12338	0.24549	0.00592		
(SD)	(0.053084)	(0.068796)	(0.115001)	(0.034117)		
Median	0.11200	-0.11140	0.21415	0.00685		
Min, Max	0.0554, 0.3100	-0.2861, -0.0354	0.1089, 0.5254	-0.0716, 0.0611		

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	26	26	14	14		
Mean	0.14003	-0.11759	0.22504	0.00379		
(SD)	(0.056288)	(0.078448)	(0.081167)	(0.049737)		
Median	0.12040	-0.11495	0.22515	-0.00125		
Min, Max	0.0682, 0.3244	-0.2995, 0.0651	0.1179, 0.4560	-0.0859, 0.0827		
Week 10						
n	23	23	13	13		
Mean	0.15201	-0.11305	0.23767	0.01995		
(SD)	(0.062522)	(0.083365)	(0.114270)	(0.038097)		
Median	0.13430	-0.10500	0.19670	0.02270		
Min, Max	0.0534, 0.3238	-0.2859, 0.0645	0.1274, 0.5817	-0.0347, 0.0855		
Week 12						
n	24	24	13	13		
Mean	0.15395	-0.11499	0.25157	0.00494		
(SD)	(0.066231)	(0.104003)	(0.118687)	(0.043105)		
Median	0.13935	-0.09660	0.22110	-0.00210		
Min, Max	0.0800, 0.3537	-0.3279, 0.0944	0.1311, 0.5375	-0.0597, 0.0948		

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	23	23	11	11		
Mean	0.14454	-0.11298	0.14589	-0.04303		
(SD)	(0.056817)	(0.093614)	(0.027418)	(0.067796)		
Median	0.14090	-0.11230	0.14430	-0.03570		
Min, Max	0.0495, 0.3100	-0.3539, 0.0012	0.0911, 0.1855	-0.1663, 0.0743		
Week 14						
n	22	22	13	13		
Mean	0.15120	-0.11339	0.12198	-0.09083		
(SD)	(0.048111)	(0.094488)	(0.035045)	(0.102437)		
Median	0.13990	-0.10800	0.11860	-0.08020		
Min, Max	0.0733, 0.2720	-0.3537, 0.0100	0.0655, 0.2053	-0.3603, 0.0290		
Week 15						
n	23	23	10	10		
Mean	0.14558	-0.09829	0.10158	-0.10414		
(SD)	(0.048370)	(0.092663)	(0.019270)	(0.051420)		
Median	0.13860	-0.08640	0.10185	-0.09915		
Min, Max	0.0736, 0.2517	-0.3686, 0.0437	0.0702, 0.1288	-0.2196, -0.0505		

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	25	25	14	14		
Mean	0.15198	-0.09850	0.12024	-0.09646		
(SD)	(0.053309)	(0.091760)	(0.057031)	(0.062981)		
Median	0.14380	-0.07070	0.10400	-0.09115		
Min, Max	0.0832, 0.2986	-0.3234, 0.0749	0.0644, 0.3029	-0.2233, 0.0193		
Week 18						
n	22	22	9	9		
Mean	0.17399	-0.08118	0.11003	-0.09518		
(SD)	(0.072032)	(0.081332)	(0.033462)	(0.054666)		
Median	0.16000	-0.10395	0.11710	-0.08230		
Min, Max	0.0761, 0.3164	-0.2201, 0.0800	0.0509, 0.1638	-0.1915, -0.0102		
Week 20						
n	25	25	8	8		
Mean	0.15482	-0.10459	0.13409	-0.09420		
(SD)	(0.060751)	(0.080138)	(0.081630)	(0.034105)		
Median	0.14590	-0.10950	0.11850	-0.08280		
Min, Max	0.0538, 0.2949	-0.3087, 0.0712	0.0547, 0.3278	-0.1504, -0.0544		

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Post-hoc Analysen Studie ALPHA

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	23	23	8	8		
Mean	0.16202	-0.09487	0.12911	-0.07280		
(SD)	(0.057596)	(0.098116)	(0.033049)	(0.025670)		
Median	0.16000	-0.10360	0.12635	-0.06795		
Min, Max	0.0708, 0.2932	-0.3880, 0.0695	0.0871, 0.1916	-0.1205, -0.0461		
Week 24						
n	25	25	8	8		
Mean	0.15215	-0.09249	0.15406	-0.09098		
(SD)	(0.065844)	(0.080589)	(0.039042)	(0.087916)		
Median	0.13830	-0.06830	0.14805	-0.06735		
Min, Max	0.0504, 0.3700	-0.3735, -0.0116	0.1016, 0.2273	-0.2964, -0.0287		
Week 28						
n	22	22	8	8	30	30
Mean	0.15833	-0.08924	0.12908	-0.07284	0.15053	-0.08486
(SD)	(0.080957)	(0.063556)	(0.019197)	(0.042575)	(0.070768)	(0.058455)
Median	0.13485	-0.08520	0.12130	-0.06395	0.12605	-0.08205
Min, Max	0.0533, 0.4700	-0.2643, -0.0148	0.1085, 0.1621	-0.1389, -0.0166	0.0533, 0.4700	-0.2643, -0.0148

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.5

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	22	22	6	6	28	28
Mean	0.15565	-0.09594	0.13510	-0.07497	0.15124	-0.09145
(SD)	(0.060996)	(0.090567)	(0.029223)	(0.059578)	(0.055907)	(0.084343)
Median	0.14630	-0.08250	0.13030	-0.07075	0.13465	-0.07635
Min, Max	0.0526, 0.2700	-0.3290, 0.0195	0.1089, 0.1900	-0.1458, 0.0100	0.0526, 0.2700	-0.3290, 0.0195
Week 36						
n	20	20	10	10	30	30
Mean	0.16645	-0.10810	0.14369	-0.07652	0.15886	-0.09757
(SD)	(0.059661)	(0.095322)	(0.050876)	(0.060695)	(0.057047)	(0.085590)
Median	0.16450	-0.10085	0.13585	-0.05470	0.15145	-0.08435
Min, Max	0.0701, 0.3384	-0.3076, 0.0860	0.0928, 0.2678	-0.2104, -0.0248	0.0701, 0.3384	-0.3076, 0.0860
Week 40						
n	20	20	7	7	27	27
Mean	0.16955	-0.07901	0.17394	-0.07443	0.17069	-0.07782
(SD)	(0.051498)	(0.082375)	(0.103339)	(0.040709)	(0.066380)	(0.073112)
Median	0.17440	-0.07765	0.13940	-0.07220	0.15330	-0.07300
Min, Max	0.0858, 0.2831	-0.2464, 0.0538	0.1109, 0.4052	-0.1474, -0.0322	0.0858, 0.4052	-0.2464, 0.0538

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.5

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	21	21	9	9	30	30
Mean	0.16608	-0.09814	0.16124	-0.06607	0.16463	-0.08852
(SD)	(0.064838)	(0.080982)	(0.064273)	(0.065242)	(0.063592)	(0.076945)
Median	0.15730	-0.09240	0.12770	-0.05460	0.15595	-0.07105
Min, Max	0.0845, 0.3216	-0.3035, 0.0464	0.1018, 0.3040	-0.1742, 0.0321	0.0845, 0.3216	-0.3035, 0.0464
Week 48						
n	17	17	7	7	24	24
Mean	0.16201	-0.09522	0.13987	-0.10850	0.15555	-0.09910
(SD)	(0.046247)	(0.068365)	(0.021485)	(0.090500)	(0.041400)	(0.073661)
Median	0.16200	-0.08630	0.13690	-0.06690	0.15050	-0.07885
Min, Max	0.1051, 0.2600	-0.2917, -0.0200	0.1148, 0.1710	-0.3072, -0.0539	0.1051, 0.2600	-0.3072, -0.0200
Week 52						
n	12	12	4	4	16	16
Mean	0.16148	-0.07138	0.14165	-0.07818	0.15652	-0.07308
(SD)	(0.042742)	(0.066112)	(0.040851)	(0.048175)	(0.041858)	(0.060652)
Median	0.14650	-0.04445	0.13465	-0.07185	0.14650	-0.06825
Min, Max	0.1143, 0.2693	-0.2386, -0.0200	0.1035, 0.1938	-0.1428, -0.0262	0.1035, 0.2693	-0.2386, -0.0200

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.5

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	20	20	6	6	26	26
Mean	0.15530	-0.10615	0.14560	-0.11093	0.15306	-0.10725
(SD)	(0.058735)	(0.087565)	(0.037810)	(0.081657)	(0.054085)	(0.084648)
Median	0.13555	-0.09140	0.13090	-0.09045	0.13420	-0.09140
Min, Max	0.0439, 0.2662	-0.3276, -0.0100	0.1234, 0.2209	-0.2573, -0.0427	0.0439, 0.2662	-0.3276, -0.0100
Week 60						
n	13	13	6	6	19	19
Mean	0.16987	-0.12088	0.13387	-0.06517	0.15850	-0.10328
(SD)	(0.056948)	(0.088157)	(0.022248)	(0.054421)	(0.050943)	(0.081925)
Median	0.16000	-0.09730	0.13050	-0.05760	0.14640	-0.09630
Min, Max	0.0908, 0.2700	-0.2899, -0.0218	0.1079, 0.1658	-0.1504, 0.0032	0.0908, 0.2700	-0.2899, 0.0032
Week 64						
n	11	11	6	6	17	17
Mean	0.17076	-0.11786	0.14298	-0.08903	0.16096	-0.10769
(SD)	(0.097005)	(0.095428)	(0.035780)	(0.106624)	(0.080428)	(0.097191)
Median	0.13740	-0.10500	0.15125	-0.05320	0.13980	-0.09440
Min, Max	0.0500, 0.3500	-0.2752, 0.0354	0.0925, 0.1808	-0.2974, -0.0173	0.0500, 0.3500	-0.2974, 0.0354

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.5

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	8	8	5	5	13	13
Mean	0.15830	-0.10290	0.17012	-0.07636	0.16285	-0.09269
(SD)	(0.063102)	(0.070419)	(0.083885)	(0.058468)	(0.068586)	(0.064906)
Median	0.14455	-0.09135	0.15160	-0.05780	0.14630	-0.07140
Min, Max	0.0815, 0.2867	-0.2357, -0.0030	0.0861, 0.3011	-0.1771, -0.0284	0.0815, 0.3011	-0.2357, -0.0030
Week 72						
n	8	8	4	4	12	12
Mean	0.16008	-0.12401	0.19503	-0.04078	0.17173	-0.09627
(SD)	(0.063951)	(0.084900)	(0.104924)	(0.063399)	(0.076819)	(0.085806)
Median	0.13990	-0.08590	0.15235	-0.02455	0.14060	-0.07620
Min, Max	0.1078, 0.3000	-0.2697, -0.0287	0.1262, 0.3492	-0.1290, 0.0150	0.1078, 0.3492	-0.2697, 0.0150
Week 80						
n	9	9	4	4	13	13
Mean	0.17710	-0.09689	0.11550	-0.12030	0.15815	-0.10409
(SD)	(0.080309)	(0.081019)	(0.049071)	(0.125535)	(0.076009)	(0.091882)
Median	0.13460	-0.09640	0.12090	-0.06050	0.13330	-0.06070
Min, Max	0.1027, 0.3500	-0.2474, 0.0200	0.0505, 0.1697	-0.3085, -0.0517	0.0505, 0.3500	-0.3085, 0.0200

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.5

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	2	2	1	1	3	3
Mean	0.16970	-0.08285	0.19700	0.02320	0.17880	-0.04750
(SD)	(0.008061)	(0.048578)	(NA)	(NA)	(0.016761)	(0.070205)
Median	0.16970	-0.08285	0.19700	0.02320	0.17540	-0.04850
Min, Max	0.1640, 0.1754	-0.1172, -0.0485	0.1970, 0.1970	0.0232, 0.0232	0.1640, 0.1970	-0.1172, 0.0232
Week 96						
n	1	1	2	2	3	3
Mean	0.17200	-0.10920	0.10645	-0.03605	0.12830	-0.06043
(SD)	(NA)	(NA)	(0.051972)	(0.007707)	(0.052753)	(0.042583)
Median	0.17200	-0.10920	0.10645	-0.03605	0.14320	-0.04150
Min, Max	0.1720, 0.1720	-0.1092, -0.1092	0.0697, 0.1432	-0.0415, -0.0306	0.0697, 0.1720	-0.1092, -0.0306
Week 104						
n	0	0	1	1	1	1
Mean			0.07600	-0.05720	0.07600	-0.05720
(SD)			(NA)	(NA)	(NA)	(NA)
Median			0.07600	-0.05720	0.07600	-0.05720
Min, Max			0.0760, 0.0760	-0.0572, -0.0572	0.0760, 0.0760	-0.0572, -0.0572

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:53:48

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.6

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		14		49	
Mean	0.25022		0.23494		0.24585	
(SD)	(0.105581)		(0.131624)		(0.112414)	
Median	0.22100		0.21555		0.22100	
Min, Max	0.1094, 0.5295		0.0394, 0.5419		0.0394, 0.5419	
Week 1						
n	28	28	12	11		
Mean	0.17800	-0.07157	0.21837	-0.00886		
(SD)	(0.058647)	(0.073036)	(0.112259)	(0.039176)		
Median	0.17280	-0.04115	0.22060	-0.00210		
Min, Max	0.0976, 0.3200	-0.2457, 0.0183	0.0410, 0.4444	-0.0975, 0.0395		
Week 2						
n	33	33	13	12		
Mean	0.13042	-0.12206	0.24298	-0.00659		
(SD)	(0.047586)	(0.112247)	(0.120948)	(0.033243)		
Median	0.12500	-0.09870	0.24360	-0.00800		
Min, Max	0.0418, 0.2299	-0.4371, 0.0089	0.0402, 0.4748	-0.0671, 0.0619		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.6

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	26	26	12	12		
Mean	0.12441	-0.11615	0.23177	-0.00640		
(SD)	(0.054368)	(0.103463)	(0.124225)	(0.046291)		
Median	0.11345	-0.09490	0.22190	-0.00355		
Min, Max	0.0500, 0.2358	-0.4571, 0.0148	0.0309, 0.5193	-0.1185, 0.0529		
Week 4						
n	33	33	11	11		
Mean	0.12968	-0.11534	0.22201	-0.00828		
(SD)	(0.053251)	(0.101456)	(0.145863)	(0.051480)		
Median	0.13020	-0.10560	0.16790	-0.00060		
Min, Max	0.0326, 0.2345	-0.3445, 0.0100	0.0388, 0.5743	-0.0993, 0.0579		
Week 6						
n	28	28	12	12		
Mean	0.14403	-0.08849	0.24249	0.00433		
(SD)	(0.057436)	(0.075937)	(0.134933)	(0.028382)		
Median	0.13575	-0.07985	0.22540	0.00465		
Min, Max	0.0646, 0.3100	-0.2861, 0.0255	0.0343, 0.5254	-0.0569, 0.0503		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.6

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	29	29	13	13		
Mean	0.14850	-0.08603	0.23257	-0.00433		
(SD)	(0.052586)	(0.084117)	(0.106251)	(0.049013)		
Median	0.14000	-0.07590	0.22750	0.00300		
Min, Max	0.0839, 0.3244	-0.2995, 0.0651	0.0424, 0.4560	-0.0859, 0.0764		
Week 10						
n	20	20	11	11		
Mean	0.17371	-0.08575	0.25655	0.00923		
(SD)	(0.057709)	(0.088513)	(0.119548)	(0.057664)		
Median	0.15510	-0.07220	0.24720	0.02270		
Min, Max	0.0900, 0.3238	-0.2859, 0.0645	0.1180, 0.5817	-0.1417, 0.0855		
Week 12						
n	29	29	13	13		
Mean	0.16550	-0.07677	0.24172	-0.00274		
(SD)	(0.062700)	(0.101954)	(0.122869)	(0.057170)		
Median	0.16780	-0.04280	0.23500	-0.00440		
Min, Max	0.0612, 0.3537	-0.3279, 0.0944	0.0383, 0.5375	-0.1279, 0.0801		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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Table GERMANY.2.6.4.6

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and \geq 8.5 g/dL)
Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	24	24	13	12		
Mean	0.16275	-0.07998	0.17556	-0.04253		
(SD)	(0.056076)	(0.101320)	(0.067054)	(0.089532)		
Median	0.15070	-0.03445	0.18230	-0.02665		
Min, Max	0.0893, 0.3100	-0.3539, 0.0460	0.0291, 0.3126	-0.2200, 0.0841		
Week 14						
n	27	27	12	12		
Mean	0.15088	-0.08622	0.13411	-0.07256		
(SD)	(0.048124)	(0.087799)	(0.048754)	(0.099002)		
Median	0.15260	-0.05280	0.13620	-0.04195		
Min, Max	0.0642, 0.2720	-0.3537, 0.0100	0.0241, 0.2053	-0.2852, 0.0501		
Week 15						
n	23	23	12	11		
Mean	0.15424	-0.07518	0.12528	-0.09311		
(SD)	(0.046359)	(0.093740)	(0.050244)	(0.097639)		
Median	0.15810	-0.05260	0.11970	-0.11150		
Min, Max	0.0880, 0.2517	-0.3686, 0.0189	0.0328, 0.2208	-0.2673, 0.0599		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.6

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	26	26	14	13		
Mean	0.16129	-0.07458	0.12299	-0.08874		
(SD)	(0.042388)	(0.089913)	(0.044632)	(0.094832)		
Median	0.16070	-0.04095	0.12940	-0.07220		
Min, Max	0.0819, 0.2540	-0.3234, 0.0240	0.0315, 0.1970	-0.2719, 0.0639		
Week 18						
n	26	26	12	11		
Mean	0.17238	-0.06479	0.14444	-0.07938		
(SD)	(0.057733)	(0.064418)	(0.061095)	(0.087311)		
Median	0.16070	-0.04855	0.13855	-0.06500		
Min, Max	0.0865, 0.3164	-0.2201, 0.0432	0.0461, 0.2377	-0.2256, 0.0396		
Week 20						
n	24	24	9	8		
Mean	0.15944	-0.09471	0.14137	-0.08320		
(SD)	(0.055906)	(0.088625)	(0.070269)	(0.080996)		
Median	0.15710	-0.06855	0.11940	-0.09625		
Min, Max	0.0838, 0.2900	-0.3087, 0.0419	0.0362, 0.2773	-0.2192, 0.0458		

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.6

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	26	26	11	10		
Mean	0.16292	-0.07372	0.14849	-0.07146		
(SD)	(0.055406)	(0.096603)	(0.062260)	(0.088043)		
Median	0.15990	-0.04745	0.13230	-0.08140		
Min, Max	0.0708, 0.2709	-0.3880, 0.0455	0.0421, 0.2625	-0.2533, 0.0531		
Week 24						
n	26	26	9	9		
Mean	0.15733	-0.07974	0.16782	-0.06527		
(SD)	(0.065438)	(0.086783)	(0.062616)	(0.100816)		
Median	0.13710	-0.05505	0.14410	-0.07340		
Min, Max	0.0351, 0.3700	-0.3735, 0.0452	0.1016, 0.2996	-0.2671, 0.0902		
Week 28						
n	24	24	8	7	32	31
Mean	0.15338	-0.07332	0.14674	-0.09143	0.15172	-0.07741
(SD)	(0.081139)	(0.059215)	(0.028616)	(0.092338)	(0.071260)	(0.066729)
Median	0.13405	-0.06195	0.15465	-0.08510	0.14280	-0.06830
Min, Max	0.0533, 0.4700	-0.2643, 0.0050	0.1085, 0.1874	-0.2632, 0.0455	0.0533, 0.4700	-0.2643, 0.0455

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.6

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	25	25	8	7	33	32
Mean	0.17114	-0.06643	0.15530	-0.07736	0.16730	-0.06882
(SD)	(0.057593)	(0.092122)	(0.061602)	(0.116986)	(0.058012)	(0.096125)
Median	0.15600	-0.03460	0.13585	-0.07280	0.15380	-0.03495
Min, Max	0.0748, 0.2757	-0.3290, 0.0531	0.0977, 0.2940	-0.2627, 0.0846	0.0748, 0.2940	-0.3290, 0.0846
Week 36						
n	22	22	7	7	29	29
Mean	0.16478	-0.08386	0.16439	-0.07371	0.16468	-0.08141
(SD)	(0.060207)	(0.093143)	(0.033211)	(0.083442)	(0.054361)	(0.089544)
Median	0.15145	-0.05145	0.16810	-0.05090	0.15240	-0.05090
Min, Max	0.0750, 0.3384	-0.3076, 0.0860	0.1294, 0.2161	-0.2388, 0.0354	0.0750, 0.3384	-0.3076, 0.0860
Week 40						
n	23	23	6	6	29	29
Mean	0.16766	-0.06214	0.16020	-0.07393	0.16611	-0.06458
(SD)	(0.050058)	(0.067438)	(0.039394)	(0.078488)	(0.047492)	(0.068535)
Median	0.17350	-0.05490	0.14940	-0.07265	0.16240	-0.05700
Min, Max	0.0839, 0.2831	-0.2464, 0.0538	0.1247, 0.2359	-0.1973, 0.0382	0.0839, 0.2831	-0.2464, 0.0538

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	21	21	9	9	30	30
Mean	0.16640	-0.07351	0.18090	-0.04689	0.17075	-0.06553
(SD)	(0.069105)	(0.075728)	(0.049685)	(0.087670)	(0.063404)	(0.078926)
Median	0.14350	-0.05630	0.18180	-0.05460	0.15595	-0.05545
Min, Max	0.0675, 0.3216	-0.3035, 0.0464	0.1271, 0.2815	-0.2264, 0.0721	0.0675, 0.3216	-0.3035, 0.0721
Week 48						
n	17	17	7	6	24	23
Mean	0.15477	-0.07679	0.16163	-0.09092	0.15677	-0.08048
(SD)	(0.046385)	(0.069835)	(0.062739)	(0.080429)	(0.050336)	(0.071114)
Median	0.13830	-0.06190	0.13230	-0.08775	0.13530	-0.06690
Min, Max	0.0903, 0.2552	-0.2917, 0.0006	0.1096, 0.2721	-0.2091, 0.0281	0.0903, 0.2721	-0.2917, 0.0281
Week 52						
n	16	16	6	5	22	21
Mean	0.15538	-0.05809	0.16075	-0.07786	0.15684	-0.06280
(SD)	(0.046178)	(0.042508)	(0.079363)	(0.058327)	(0.055034)	(0.045935)
Median	0.14770	-0.04305	0.12385	-0.07340	0.14600	-0.07030
Min, Max	0.0871, 0.2693	-0.1159, -0.0029	0.1035, 0.3085	-0.1337, 0.0128	0.0871, 0.3085	-0.1337, 0.0128

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ALXN2040-PNH-301

Table GERMANY.2.6.4.6

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	19	19	6	5	25	24
Mean	0.16494	-0.08482	0.16560	-0.09438	0.16510	-0.08681
(SD)	(0.057991)	(0.086844)	(0.054707)	(0.047737)	(0.056088)	(0.079463)
Median	0.17670	-0.04290	0.15420	-0.07790	0.16490	-0.06755
Min, Max	0.0736, 0.2662	-0.3276, -0.0079	0.1234, 0.2705	-0.1627, -0.0504	0.0736, 0.2705	-0.3276, -0.0079
Week 60						
n	14	14	6	6	20	20
Mean	0.17314	-0.09327	0.17480	-0.05933	0.17364	-0.08309
(SD)	(0.056576)	(0.086079)	(0.070993)	(0.061414)	(0.059304)	(0.079479)
Median	0.16290	-0.07390	0.16250	-0.06570	0.16250	-0.06570
Min, Max	0.0906, 0.2700	-0.2899, -0.0025	0.1157, 0.3121	-0.1211, 0.0550	0.0906, 0.3121	-0.2899, 0.0550
Week 64						
n	14	14	7	7	21	21
Mean	0.17260	-0.08039	0.15447	-0.06210	0.16656	-0.07429
(SD)	(0.083663)	(0.078174)	(0.065794)	(0.054222)	(0.076974)	(0.070230)
Median	0.14670	-0.07370	0.13980	-0.07240	0.14340	-0.07240
Min, Max	0.0783, 0.3500	-0.2592, 0.0354	0.0925, 0.2906	-0.1426, 0.0185	0.0783, 0.3500	-0.2592, 0.0354

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Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	10	10	4	4	14	14
Mean	0.15712	-0.07326	0.14580	-0.05168	0.15389	-0.06709
(SD)	(0.073144)	(0.038177)	(0.035729)	(0.051008)	(0.063456)	(0.041375)
Median	0.14105	-0.07070	0.13570	-0.06460	0.13585	-0.06915
Min, Max	0.0637, 0.2867	-0.1328, -0.0030	0.1160, 0.1958	-0.0981, 0.0206	0.0637, 0.2867	-0.1328, 0.0206
Week 72						
n	9	9	5	5	14	14
Mean	0.15804	-0.08282	0.18070	-0.03272	0.16614	-0.06493
(SD)	(0.062063)	(0.061204)	(0.100341)	(0.061899)	(0.074801)	(0.064069)
Median	0.14980	-0.07410	0.13830	-0.04240	0.14315	-0.07285
Min, Max	0.0932, 0.3000	-0.2295, -0.0287	0.1262, 0.3596	-0.1064, 0.0438	0.0932, 0.3596	-0.2295, 0.0438
Week 80						
n	7	7	5	5	12	12
Mean	0.17009	-0.08371	0.11676	-0.09666	0.14787	-0.08911
(SD)	(0.095354)	(0.059200)	(0.037977)	(0.122436)	(0.078980)	(0.086065)
Median	0.12390	-0.09640	0.12840	-0.06070	0.12615	-0.07855
Min, Max	0.0829, 0.3500	-0.1795, -0.0087	0.0505, 0.1418	-0.2922, 0.0376	0.0505, 0.3500	-0.2922, 0.0376

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Post-hoc Analysen Studie ALPHA

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Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	4	4	3	3	7	7
Mean	0.16123	-0.06135	0.22537	-0.05853	0.18871	-0.06014
(SD)	(0.033753)	(0.053712)	(0.048097)	(0.088367)	(0.050162)	(0.063621)
Median	0.16970	-0.06775	0.19820	-0.04650	0.19190	-0.04850
Min, Max	0.1136, 0.1919	-0.1172, 0.0073	0.1970, 0.2809	-0.1523, 0.0232	0.1136, 0.2809	-0.1523, 0.0232
Week 96						
n	3	3	4	4	7	7
Mean	0.16340	-0.05737	0.11378	-0.12695	0.13504	-0.09713
(SD)	(0.013869)	(0.045256)	(0.032778)	(0.136454)	(0.036124)	(0.106658)
Median	0.17080	-0.03720	0.12110	-0.07635	0.14320	-0.04150
Min, Max	0.1474, 0.1720	-0.1092, -0.0257	0.0697, 0.1432	-0.3245, -0.0306	0.0697, 0.1720	-0.3245, -0.0257
Week 104						
n	0	0	1	1	1	1
Mean			0.18960	-0.05510	0.18960	-0.05510
(SD)			(NA)	(NA)	(NA)	(NA)
Median			0.18960	-0.05510	0.18960	-0.05510
Min, Max			0.1896, 0.1896	-0.0551, -0.0551	0.1896, 0.1896	-0.0551, -0.0551

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.6

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		11		33	
Mean	0.24348		0.19830		0.22842	
(SD)	(0.084319)		(0.101428)		(0.091369)	
Median	0.21680		0.17310		0.20000	
Min, Max	0.1357, 0.4400		0.1200, 0.4782		0.1200, 0.4782	
Week 1						
n	19	19	9	9		
Mean	0.15234	-0.08693	0.19378	0.02121		
(SD)	(0.060757)	(0.046960)	(0.067685)	(0.039044)		
Median	0.15690	-0.08570	0.21430	0.02000		
Min, Max	0.0553, 0.2864	-0.2100, -0.0222	0.1015, 0.2964	-0.0473, 0.0929		
Week 2						
n	20	20	9	9		
Mean	0.11286	-0.13549	0.18900	0.01643		
(SD)	(0.067442)	(0.075614)	(0.046285)	(0.027212)		
Median	0.09655	-0.11975	0.20180	0.01720		
Min, Max	0.0388, 0.3474	-0.3609, -0.0596	0.1257, 0.2775	-0.0231, 0.0686		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	20	20	8	8		
Mean	0.10374	-0.13356	0.17746	-0.00043		
(SD)	(0.058821)	(0.078564)	(0.049144)	(0.019246)		
Median	0.08785	-0.12775	0.18210	-0.00225		
Min, Max	0.0400, 0.2506	-0.4000, -0.0247	0.1013, 0.2476	-0.0319, 0.0283		
Week 4						
n	17	17	10	10		
Mean	0.10927	-0.12271	0.19252	-0.01061		
(SD)	(0.058276)	(0.083099)	(0.073985)	(0.042117)		
Median	0.08770	-0.12590	0.19340	0.00560		
Min, Max	0.0525, 0.2846	-0.3016, 0.1083	0.1071, 0.3644	-0.1138, 0.0225		
Week 6						
n	19	19	10	10		
Mean	0.10863	-0.12670	0.19836	-0.00477		
(SD)	(0.032875)	(0.070751)	(0.092686)	(0.039713)		
Median	0.10420	-0.11590	0.20390	-0.00150		
Min, Max	0.0554, 0.1775	-0.3016, -0.0399	0.1000, 0.4066	-0.0716, 0.0611		

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Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	15	15	9	9		
Mean	0.11603	-0.11031	0.17641	0.00384		
(SD)	(0.037329)	(0.052154)	(0.052140)	(0.037927)		
Median	0.10490	-0.11220	0.17240	-0.00400		
Min, Max	0.0682, 0.1978	-0.2102, -0.0276	0.1000, 0.2627	-0.0300, 0.0827		
Week 10						
n	17	17	9	9		
Mean	0.13172	-0.10844	0.18028	0.00771		
(SD)	(0.043240)	(0.060412)	(0.052574)	(0.035515)		
Median	0.13180	-0.10490	0.16000	-0.00030		
Min, Max	0.0534, 0.2219	-0.2202, -0.0260	0.1274, 0.2724	-0.0347, 0.0826		
Week 12						
n	16	16	9	9		
Mean	0.13196	-0.11981	0.20718	0.01018		
(SD)	(0.040202)	(0.074945)	(0.103290)	(0.038624)		
Median	0.12860	-0.10270	0.16720	0.01080		
Min, Max	0.0800, 0.2385	-0.3000, -0.0089	0.1000, 0.4441	-0.0341, 0.0948		

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Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	14	14	7	7		
Mean	0.13221	-0.10575	0.14074	-0.03251		
(SD)	(0.044253)	(0.075536)	(0.037083)	(0.049741)		
Median	0.13895	-0.09635	0.14430	-0.03570		
Min, Max	0.0495, 0.2000	-0.2800, 0.0000	0.0911, 0.1872	-0.1225, 0.0300		
Week 14						
n	13	13	10	10		
Mean	0.14411	-0.12124	0.10953	-0.09360		
(SD)	(0.042783)	(0.087824)	(0.027272)	(0.103584)		
Median	0.14280	-0.11040	0.11825	-0.08100		
Min, Max	0.0804, 0.2018	-0.2591, 0.0018	0.0636, 0.1468	-0.3603, 0.0100		
Week 15						
n	16	16	7	7		
Mean	0.13936	-0.09478	0.10566	-0.07781		
(SD)	(0.047562)	(0.083821)	(0.019528)	(0.035335)		
Median	0.12550	-0.08160	0.09850	-0.08510		
Min, Max	0.0736, 0.2437	-0.2845, 0.0437	0.0827, 0.1321	-0.1295, -0.0400		

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Post-hoc Analysen Studie ALPHA

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Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	16	16	9	9		
Mean	0.14334	-0.09610	0.11662	-0.08984		
(SD)	(0.054039)	(0.077073)	(0.073454)	(0.047293)		
Median	0.14645	-0.08755	0.10000	-0.08930		
Min, Max	0.0832, 0.2986	-0.2463, 0.0749	0.0516, 0.3029	-0.1753, -0.0200		
Week 18						
n	13	13	6	6		
Mean	0.16253	-0.07041	0.10413	-0.07082		
(SD)	(0.068662)	(0.095323)	(0.035850)	(0.053140)		
Median	0.16380	-0.07890	0.10930	-0.07090		
Min, Max	0.0761, 0.2993	-0.2148, 0.0800	0.0509, 0.1548	-0.1568, 0.0060		
Week 20						
n	17	17	8	8		
Mean	0.14729	-0.08604	0.13154	-0.08109		
(SD)	(0.059352)	(0.066717)	(0.083211)	(0.039395)		
Median	0.14120	-0.08720	0.12020	-0.06950		
Min, Max	0.0538, 0.2949	-0.2072, 0.0712	0.0547, 0.3278	-0.1504, -0.0487		

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Post-hoc Analysen Studie ALPHA

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ALXN2040-PNH-301

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Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	14	14	6	6		
Mean	0.15111	-0.09296	0.11382	-0.06832		
(SD)	(0.053216)	(0.080083)	(0.029477)	(0.027633)		
Median	0.14160	-0.10880	0.11730	-0.05925		
Min, Max	0.0727, 0.2932	-0.2762, 0.0695	0.0729, 0.1505	-0.1205, -0.0461		
Week 24						
n	16	16	5	5		
Mean	0.13313	-0.08960	0.16370	-0.08924		
(SD)	(0.049598)	(0.063189)	(0.015557)	(0.123587)		
Median	0.13415	-0.07160	0.16570	-0.05460		
Min, Max	0.0504, 0.2444	-0.2724, -0.0222	0.1448, 0.1818	-0.2964, 0.0261		
Week 28						
n	15	15	6	6	21	21
Mean	0.14647	-0.09583	0.12177	-0.06037	0.13941	-0.08570
(SD)	(0.028535)	(0.070491)	(0.019668)	(0.043896)	(0.028240)	(0.065034)
Median	0.14080	-0.08720	0.11800	-0.05000	0.13540	-0.07630
Min, Max	0.1131, 0.2121	-0.2564, -0.0159	0.0968, 0.1496	-0.1389, -0.0166	0.0968, 0.2121	-0.2564, -0.0159

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Post-hoc Analysen Studie ALPHA

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Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	15	15	6	6	21	21
Mean	0.14176	-0.09934	0.11785	-0.06375	0.13493	-0.08917
(SD)	(0.051759)	(0.081785)	(0.041423)	(0.051093)	(0.049262)	(0.074875)
Median	0.13970	-0.08510	0.11375	-0.06340	0.13070	-0.06870
Min, Max	0.0526, 0.2357	-0.3003, 0.0000	0.0689, 0.1900	-0.1458, 0.0100	0.0526, 0.2357	-0.3003, 0.0100
Week 36						
n	11	11	9	9	20	20
Mean	0.14050	-0.10269	0.13126	-0.08000	0.13634	-0.09248
(SD)	(0.040005)	(0.058470)	(0.058385)	(0.062319)	(0.047957)	(0.059738)
Median	0.15000	-0.10210	0.10410	-0.06160	0.13015	-0.07540
Min, Max	0.0701, 0.1849	-0.2087, -0.0200	0.0734, 0.2678	-0.2104, -0.0248	0.0701, 0.2678	-0.2104, -0.0200
Week 40						
n	12	12	5	5	17	17
Mean	0.15096	-0.10913	0.16956	-0.08338	0.15643	-0.10155
(SD)	(0.042226)	(0.095250)	(0.135989)	(0.041913)	(0.076977)	(0.082600)
Median	0.14865	-0.09205	0.12720	-0.07300	0.14150	-0.08980
Min, Max	0.0858, 0.2200	-0.2646, 0.0200	0.0567, 0.4052	-0.1474, -0.0322	0.0567, 0.4052	-0.2646, 0.0200

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Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	13	13	5	5	18	18
Mean	0.15189	-0.10354	0.16170	-0.08812	0.15462	-0.09926
(SD)	(0.040981)	(0.071970)	(0.084145)	(0.072594)	(0.053590)	(0.070333)
Median	0.16100	-0.10990	0.12390	-0.07550	0.15625	-0.10975
Min, Max	0.0845, 0.2089	-0.2311, -0.0200	0.1018, 0.3040	-0.1742, -0.0085	0.0845, 0.3040	-0.2311, -0.0085
Week 48						
n	12	12	5	5	17	17
Mean	0.17755	-0.08470	0.13292	-0.12488	0.16442	-0.09652
(SD)	(0.055084)	(0.062268)	(0.034927)	(0.104031)	(0.053202)	(0.075679)
Median	0.18010	-0.08525	0.13690	-0.09430	0.15330	-0.09430
Min, Max	0.1051, 0.2753	-0.2018, 0.0105	0.0788, 0.1710	-0.3072, -0.0554	0.0788, 0.2753	-0.3072, 0.0105
Week 52						
n	7	7	4	4	11	11
Mean	0.14829	-0.11754	0.10578	-0.07958	0.13283	-0.10374
(SD)	(0.040997)	(0.087789)	(0.039327)	(0.048090)	(0.043960)	(0.075398)
Median	0.14340	-0.11510	0.10465	-0.07465	0.13680	-0.07930
Min, Max	0.0764, 0.1989	-0.2386, -0.0200	0.0600, 0.1538	-0.1428, -0.0262	0.0600, 0.1989	-0.2386, -0.0200

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.6

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	10	10	4	4	14	14
Mean	0.14395	-0.10008	0.14055	-0.12108	0.14298	-0.10608
(SD)	(0.057268)	(0.064977)	(0.058898)	(0.099877)	(0.055440)	(0.072951)
Median	0.12530	-0.09140	0.13065	-0.09215	0.12590	-0.09140
Min, Max	0.0439, 0.2274	-0.2592, -0.0100	0.0800, 0.2209	-0.2573, -0.0427	0.0439, 0.2274	-0.2592, -0.0100
Week 60						
n	8	8	5	5	13	13
Mean	0.15568	-0.09051	0.12560	-0.04932	0.14411	-0.07467
(SD)	(0.048946)	(0.068458)	(0.018942)	(0.059860)	(0.041821)	(0.066055)
Median	0.15320	-0.07180	0.12090	-0.02720	0.14330	-0.05220
Min, Max	0.0908, 0.2348	-0.2387, -0.0247	0.1079, 0.1528	-0.1504, 0.0032	0.0908, 0.2348	-0.2387, 0.0032
Week 64						
n	5	5	4	4	9	9
Mean	0.10388	-0.14646	0.12608	-0.11425	0.11374	-0.13214
(SD)	(0.031318)	(0.082794)	(0.058756)	(0.124920)	(0.043839)	(0.097813)
Median	0.11100	-0.15000	0.13675	-0.07115	0.11100	-0.08220
Min, Max	0.0500, 0.1259	-0.2752, -0.0656	0.0500, 0.1808	-0.2974, -0.0173	0.0500, 0.1808	-0.2974, -0.0173

Baseline is defined as the last non-missing value prior to the first dose of study drug.
 Change from baseline is summarized only for patients who have data at baseline and the specified time point.
 Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
 D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
 Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.6

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	5	5	5	5	10	10
Mean	0.12842	-0.14348	0.13624	-0.08266	0.13233	-0.11307
(SD)	(0.026681)	(0.063825)	(0.099106)	(0.057312)	(0.068547)	(0.065558)
Median	0.13820	-0.14680	0.09240	-0.08000	0.13575	-0.09960
Min, Max	0.0815, 0.1463	-0.2357, -0.0599	0.0500, 0.3011	-0.1771, -0.0284	0.0500, 0.3011	-0.2357, -0.0284
Week 72						
n	5	5	3	3	8	8
Mean	0.21228	-0.10898	0.20857	-0.06853	0.21089	-0.09381
(SD)	(0.117290)	(0.104239)	(0.126735)	(0.061161)	(0.111597)	(0.087840)
Median	0.15100	-0.07000	0.17330	-0.06990	0.16215	-0.06995
Min, Max	0.1123, 0.3833	-0.2697, -0.0002	0.1032, 0.3492	-0.1290, -0.0067	0.1032, 0.3833	-0.2697, -0.0002
Week 80						
n	4	4	2	2	6	6
Mean	0.16553	-0.10310	0.14470	-0.18440	0.15858	-0.13020
(SD)	(0.040978)	(0.113029)	(0.035355)	(0.175504)	(0.037056)	(0.124853)
Median	0.15440	-0.09250	0.14470	-0.18440	0.15215	-0.09270
Min, Max	0.1333, 0.2200	-0.2474, 0.0200	0.1197, 0.1697	-0.3085, -0.0603	0.1197, 0.2200	-0.3085, 0.0200

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.6

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	2	2	0	0	2	2
Mean	0.14715	-0.21535			0.14715	-0.21535
(SD)	(0.096661)	(0.206263)			(0.096661)	(0.206263)
Median	0.14715	-0.21535			0.14715	-0.21535
Min, Max	0.0788, 0.2155	-0.3612, -0.0695			0.0788, 0.2155	-0.3612, -0.0695
Week 96						
n	1	1	0	0	1	1
Mean	0.18940	-0.09560			0.18940	-0.09560
(SD)	(NA)	(NA)			(NA)	(NA)
Median	0.18940	-0.09560			0.18940	-0.09560
Min, Max	0.1894, 0.1894	-0.0956, -0.0956			0.1894, 0.1894	-0.0956, -0.0956
Week 104						
n	1	1	1	1	2	2
Mean	0.17020	-0.11480	0.07600	-0.05720	0.12310	-0.08600
(SD)	(NA)	(NA)	(NA)	(NA)	(0.066609)	(0.040729)
Median	0.17020	-0.11480	0.07600	-0.05720	0.12310	-0.08600
Min, Max	0.1702, 0.1702	-0.1148, -0.1148	0.0760, 0.0760	-0.0572, -0.0572	0.0760, 0.1702	-0.1148, -0.0572

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

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Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		11		32	
Mean	0.23724		0.22068		0.23155	
(SD)	(0.069860)		(0.105414)		(0.082444)	
Median	0.22390		0.17400		0.21195	
Min, Max	0.1094, 0.3820		0.1112, 0.4782		0.1094, 0.4782	
Week 1						
n	16	16	8	7		
Mean	0.16646	-0.06364	0.21906	0.01303		
(SD)	(0.053545)	(0.055552)	(0.091816)	(0.028130)		
Median	0.16005	-0.04435	0.19460	-0.00210		
Min, Max	0.0553, 0.2690	-0.2067, 0.0183	0.1200, 0.3962	-0.0164, 0.0499		
Week 2						
n	20	20	9	8		
Mean	0.11992	-0.12123	0.21990	0.00118		
(SD)	(0.044967)	(0.079500)	(0.085811)	(0.015140)		
Median	0.11520	-0.10760	0.16930	-0.00565		
Min, Max	0.0388, 0.1934	-0.3258, -0.0202	0.1367, 0.3798	-0.0165, 0.0255		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	15	15	7	7		
Mean	0.12773	-0.10049	0.21276	0.00420		
(SD)	(0.052112)	(0.058925)	(0.071213)	(0.028233)		
Median	0.12350	-0.10340	0.18680	-0.01070		
Min, Max	0.0534, 0.2479	-0.2000, -0.0030	0.1519, 0.3531	-0.0219, 0.0529		
Week 4						
n	19	19	8	8		
Mean	0.12585	-0.11552	0.19475	-0.02316		
(SD)	(0.062282)	(0.105416)	(0.078939)	(0.041421)		
Median	0.11140	-0.10940	0.16855	-0.01600		
Min, Max	0.0326, 0.2846	-0.3350, 0.1083	0.1280, 0.3644	-0.1138, 0.0168		
Week 6						
n	15	15	9	9		
Mean	0.13300	-0.09619	0.23572	0.00593		
(SD)	(0.038519)	(0.068316)	(0.102100)	(0.037986)		
Median	0.13000	-0.07990	0.20410	0.02660		
Min, Max	0.0649, 0.1881	-0.2521, 0.0035	0.1000, 0.4066	-0.0716, 0.0503		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	16	16	8	8		
Mean	0.14801	-0.08457	0.19493	-0.00381		
(SD)	(0.060158)	(0.068970)	(0.058730)	(0.040806)		
Median	0.13315	-0.09260	0.19980	-0.01055		
Min, Max	0.0839, 0.3244	-0.2102, 0.0651	0.1000, 0.2644	-0.0604, 0.0764		
Week 10						
n	12	12	9	9		
Mean	0.15100	-0.09317	0.21471	0.01479		
(SD)	(0.063781)	(0.072270)	(0.054005)	(0.033590)		
Median	0.13900	-0.09190	0.19670	0.01000		
Min, Max	0.0534, 0.3238	-0.2202, 0.0645	0.1400, 0.2930	-0.0318, 0.0855		
Week 12						
n	12	12	8	8		
Mean	0.16361	-0.06503	0.22879	-0.00971		
(SD)	(0.073854)	(0.083925)	(0.111040)	(0.038054)		
Median	0.17190	-0.05775	0.20140	-0.02155		
Min, Max	0.0612, 0.3537	-0.2054, 0.0944	0.1000, 0.4441	-0.0341, 0.0801		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	9	9	9	8		
Mean	0.13398	-0.07611	0.19382	-0.02969		
(SD)	(0.048192)	(0.072138)	(0.055088)	(0.078632)		
Median	0.14090	-0.05840	0.18550	-0.01375		
Min, Max	0.0495, 0.2138	-0.2184, 0.0025	0.1358, 0.3126	-0.1663, 0.0743		
Week 14						
n	12	12	10	10		
Mean	0.15131	-0.08249	0.13625	-0.09150		
(SD)	(0.036190)	(0.069518)	(0.027677)	(0.118837)		
Median	0.14780	-0.05275	0.12550	-0.04690		
Min, Max	0.0914, 0.2066	-0.2591, -0.0180	0.1063, 0.1988	-0.3603, 0.0290		
Week 15						
n	13	13	9	8		
Mean	0.14714	-0.08214	0.13234	-0.08198		
(SD)	(0.041838)	(0.068648)	(0.038203)	(0.072807)		
Median	0.15990	-0.07680	0.12300	-0.05420		
Min, Max	0.0891, 0.2132	-0.2572, 0.0019	0.0900, 0.2208	-0.2196, 0.0114		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	15	15	10	9		
Mean	0.15540	-0.07561	0.13705	-0.09580		
(SD)	(0.036205)	(0.060241)	(0.063458)	(0.080736)		
Median	0.15670	-0.05920	0.12250	-0.07220		
Min, Max	0.0848, 0.2184	-0.2382, -0.0129	0.0891, 0.3029	-0.2233, 0.0193		
Week 18						
n	13	13	8	7		
Mean	0.15882	-0.05895	0.14195	-0.08816		
(SD)	(0.054445)	(0.067605)	(0.058643)	(0.079143)		
Median	0.16520	-0.03160	0.12585	-0.05540		
Min, Max	0.0761, 0.2892	-0.2148, 0.0299	0.0800, 0.2377	-0.1915, 0.0050		
Week 20						
n	14	14	7	6		
Mean	0.13858	-0.09611	0.16694	-0.09447		
(SD)	(0.044895)	(0.072507)	(0.095030)	(0.048111)		
Median	0.14265	-0.08770	0.12440	-0.09250		
Min, Max	0.0538, 0.2005	-0.2686, -0.0063	0.0800, 0.3278	-0.1504, -0.0487		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	13	13	6	5		
Mean	0.16030	-0.07041	0.15748	-0.06504		
(SD)	(0.056973)	(0.049219)	(0.064693)	(0.077140)		
Median	0.16300	-0.08390	0.12910	-0.05820		
Min, Max	0.0727, 0.2537	-0.1293, 0.0127	0.0985, 0.2625	-0.1462, 0.0531		
Week 24						
n	14	14	5	5		
Mean	0.14304	-0.06667	0.18394	-0.08894		
(SD)	(0.062474)	(0.044965)	(0.067778)	(0.140887)		
Median	0.15075	-0.06165	0.16570	-0.09260		
Min, Max	0.0351, 0.2444	-0.1259, 0.0011	0.1285, 0.2996	-0.2964, 0.0902		
Week 28						
n	13	13	5	4	18	17
Mean	0.14251	-0.07805	0.13728	-0.08773	0.14106	-0.08032
(SD)	(0.032958)	(0.057820)	(0.035940)	(0.037124)	(0.032810)	(0.052761)
Median	0.12600	-0.06820	0.12320	-0.08070	0.12520	-0.07630
Min, Max	0.0932, 0.1899	-0.2272, -0.0051	0.0968, 0.1874	-0.1389, -0.0506	0.0932, 0.1899	-0.2272, -0.0051

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:53:53

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	13	13	7	6	20	19
Mean	0.16462	-0.05262	0.14536	-0.05692	0.15788	-0.05398
(SD)	(0.054741)	(0.048228)	(0.070793)	(0.085792)	(0.059700)	(0.059995)
Median	0.14990	-0.03660	0.11500	-0.04905	0.14450	-0.04000
Min, Max	0.0748, 0.2487	-0.1463, 0.0260	0.0900, 0.2940	-0.1470, 0.0846	0.0748, 0.2940	-0.1470, 0.0846
Week 36						
n	12	12	5	5	17	17
Mean	0.15512	-0.08316	0.15458	-0.11104	0.15496	-0.09136
(SD)	(0.039510)	(0.054095)	(0.068544)	(0.069064)	(0.047411)	(0.058101)
Median	0.16450	-0.06950	0.12940	-0.07660	0.16030	-0.07540
Min, Max	0.0701, 0.2190	-0.2087, -0.0264	0.1035, 0.2678	-0.2104, -0.0444	0.0701, 0.2678	-0.2104, -0.0264
Week 40						
n	13	13	4	4	17	17
Mean	0.15872	-0.07222	0.20298	-0.08578	0.16914	-0.07541
(SD)	(0.044489)	(0.061455)	(0.136117)	(0.046928)	(0.073026)	(0.057276)
Median	0.15330	-0.05490	0.14790	-0.08065	0.15330	-0.07300
Min, Max	0.0839, 0.2184	-0.2405, -0.0052	0.1109, 0.4052	-0.1474, -0.0344	0.0839, 0.4052	-0.2405, -0.0052

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	13	13	6	6	19	19
Mean	0.16268	-0.07596	0.19880	-0.05760	0.17409	-0.07016
(SD)	(0.060402)	(0.069827)	(0.080560)	(0.097731)	(0.067324)	(0.077334)
Median	0.15460	-0.05980	0.18615	-0.06230	0.15730	-0.05980
Min, Max	0.0675, 0.3057	-0.2305, 0.0464	0.1073, 0.3040	-0.1742, 0.0721	0.0675, 0.3057	-0.2305, 0.0721
Week 48						
n	11	11	6	5	17	16
Mean	0.18325	-0.06526	0.15078	-0.13910	0.17179	-0.08834
(SD)	(0.037549)	(0.059399)	(0.067771)	(0.098365)	(0.050718)	(0.078624)
Median	0.18020	-0.06190	0.13660	-0.10500	0.17100	-0.07885
Min, Max	0.1253, 0.2600	-0.2018, 0.0105	0.0788, 0.2721	-0.3072, -0.0539	0.0788, 0.2721	-0.3072, 0.0105
Week 52						
n	9	9	6	5	15	14
Mean	0.17059	-0.06397	0.10242	-0.09922	0.14332	-0.07656
(SD)	(0.051132)	(0.077345)	(0.024164)	(0.035969)	(0.053829)	(0.066233)
Median	0.17420	-0.02270	0.10725	-0.07930	0.14340	-0.07015
Min, Max	0.0764, 0.2693	-0.2386, -0.0029	0.0600, 0.1307	-0.1428, -0.0700	0.0600, 0.2693	-0.2386, -0.0029

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	9	9	6	5	15	14
Mean	0.18574	-0.07411	0.14667	-0.11398	0.17011	-0.08835
(SD)	(0.044796)	(0.079143)	(0.048473)	(0.087165)	(0.048770)	(0.081150)
Median	0.18600	-0.03970	0.14445	-0.07790	0.17670	-0.05965
Min, Max	0.1228, 0.2662	-0.2592, -0.0079	0.0800, 0.2209	-0.2573, -0.0500	0.0800, 0.2662	-0.2592, -0.0079
Week 60						
n	8	8	5	5	13	13
Mean	0.17056	-0.07224	0.12518	-0.07080	0.15311	-0.07168
(SD)	(0.042479)	(0.077017)	(0.026332)	(0.048555)	(0.042566)	(0.065165)
Median	0.17050	-0.04135	0.11570	-0.05810	0.15160	-0.05220
Min, Max	0.0974, 0.2375	-0.2387, -0.0025	0.1079, 0.1714	-0.1504, -0.0200	0.0974, 0.2375	-0.2387, -0.0025
Week 64						
n	6	6	6	6	12	12
Mean	0.17935	-0.07737	0.12125	-0.09725	0.15030	-0.08731
(SD)	(0.079504)	(0.108233)	(0.046796)	(0.101772)	(0.069203)	(0.100700)
Median	0.15745	-0.05575	0.12530	-0.07115	0.13925	-0.06395
Min, Max	0.1068, 0.3251	-0.2752, 0.0354	0.0500, 0.1808	-0.2974, -0.0187	0.0500, 0.3251	-0.2974, 0.0354

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB
Run Date: 2024-04-26T15:53:53

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	6	6	5	5	11	11
Mean	0.18168	-0.08043	0.14122	-0.09874	0.16329	-0.08875
(SD)	(0.058132)	(0.080202)	(0.096087)	(0.046080)	(0.076350)	(0.064474)
Median	0.16265	-0.06320	0.11600	-0.08070	0.14660	-0.07450
Min, Max	0.1251, 0.2867	-0.2357, -0.0030	0.0500, 0.3011	-0.1771, -0.0578	0.0500, 0.3011	-0.2357, -0.0030
Week 72						
n	4	4	5	5	9	9
Mean	0.15523	-0.11270	0.16966	-0.06654	0.16324	-0.08706
(SD)	(0.037172)	(0.107049)	(0.101227)	(0.056420)	(0.075495)	(0.080503)
Median	0.15285	-0.07620	0.13140	-0.06990	0.13830	-0.07410
Min, Max	0.1123, 0.2029	-0.2697, -0.0287	0.1032, 0.3492	-0.1290, 0.0150	0.1032, 0.3492	-0.2697, 0.0150
Week 80						
n	4	4	4	4	8	8
Mean	0.16643	-0.10150	0.11768	-0.13430	0.14205	-0.11790
(SD)	(0.055121)	(0.105788)	(0.049510)	(0.119596)	(0.055061)	(0.105988)
Median	0.15525	-0.07495	0.12525	-0.08850	0.13150	-0.08465
Min, Max	0.1153, 0.2399	-0.2474, -0.0087	0.0505, 0.1697	-0.3085, -0.0517	0.0505, 0.2399	-0.3085, -0.0087

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	3	3	2	2	5	5
Mean	0.17710	-0.05280	0.19760	-0.01165	0.18530	-0.03634
(SD)	(0.014027)	(0.062361)	(0.000849)	(0.049285)	(0.014988)	(0.055315)
Median	0.17540	-0.04850	0.19760	-0.01165	0.19190	-0.04650
Min, Max	0.1640, 0.1919	-0.1172, 0.0073	0.1970, 0.1982	-0.0465, 0.0232	0.1640, 0.1982	-0.1172, 0.0232
Week 96						
n	2	2	3	3	5	5
Mean	0.15970	-0.07320	0.11547	-0.06110	0.13316	-0.06594
(SD)	(0.017395)	(0.050912)	(0.039931)	(0.043729)	(0.038208)	(0.040596)
Median	0.15970	-0.07320	0.13350	-0.04150	0.14320	-0.04150
Min, Max	0.1474, 0.1720	-0.1092, -0.0372	0.0697, 0.1432	-0.1112, -0.0306	0.0697, 0.1720	-0.1112, -0.0306
Week 104						
n	0	0	1	1	1	1
Mean			0.18960	-0.05510	0.18960	-0.05510
(SD)			(NA)	(NA)	(NA)	(NA)
Median			0.18960	-0.05510	0.18960	-0.05510
Min, Max			0.1896, 0.1896	-0.0551, -0.0551	0.1896, 0.1896	-0.0551, -0.0551

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		14		50	
Mean	0.25367		0.21735		0.24350	
(SD)	(0.110550)		(0.131610)		(0.116603)	
Median	0.22065		0.19055		0.21010	
Min, Max	0.1200, 0.5295		0.0394, 0.5419		0.0394, 0.5419	
Week 1						
n	31	31	13	13		
Mean	0.16822	-0.08508	0.20092	0.00017		
(SD)	(0.064220)	(0.067143)	(0.099054)	(0.047010)		
Median	0.15710	-0.08250	0.21680	0.00650		
Min, Max	0.0639, 0.3200	-0.2457, 0.0100	0.0410, 0.4444	-0.0975, 0.0929		
Week 2						
n	33	33	13	13		
Mean	0.12615	-0.13070	0.22158	0.00457		
(SD)	(0.062205)	(0.110800)	(0.111523)	(0.039942)		
Median	0.11970	-0.09880	0.20680	0.00080		
Min, Max	0.0419, 0.3474	-0.4371, 0.0089	0.0402, 0.4748	-0.0671, 0.0686		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	31	31	13	13		
Mean	0.10947	-0.13496	0.20858	-0.00843		
(SD)	(0.058616)	(0.104514)	(0.119130)	(0.041660)		
Median	0.08950	-0.11520	0.20830	0.00140		
Min, Max	0.0400, 0.2506	-0.4571, 0.0148	0.0309, 0.5193	-0.1185, 0.0443		
Week 4						
n	31	31	13	13		
Mean	0.12083	-0.11927	0.21610	-0.00092		
(SD)	(0.051520)	(0.089497)	(0.135617)	(0.048334)		
Median	0.11050	-0.11950	0.20250	0.01660		
Min, Max	0.0529, 0.2345	-0.3445, 0.0100	0.0388, 0.5743	-0.0993, 0.0579		
Week 6						
n	32	32	13	13		
Mean	0.12818	-0.10756	0.21323	-0.00378		
(SD)	(0.057278)	(0.079447)	(0.129785)	(0.030903)		
Median	0.11140	-0.09505	0.20810	-0.00410		
Min, Max	0.0554, 0.3100	-0.3016, 0.0255	0.0343, 0.5254	-0.0569, 0.0611		

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	28	28	14	14		
Mean	0.13138	-0.09987	0.21798	0.00063		
(SD)	(0.043143)	(0.078949)	(0.106389)	(0.047177)		
Median	0.12040	-0.08705	0.20910	0.00120		
Min, Max	0.0682, 0.2300	-0.2995, 0.0106	0.0424, 0.4560	-0.0859, 0.0827		
Week 10						
n	25	25	11	11		
Mean	0.15605	-0.09762	0.22838	0.00344		
(SD)	(0.051816)	(0.080200)	(0.130219)	(0.058042)		
Median	0.14850	-0.07110	0.19180	0.01380		
Min, Max	0.0768, 0.2541	-0.2859, 0.0068	0.1180, 0.5817	-0.1417, 0.0826		
Week 12						
n	33	33	14	14		
Mean	0.14992	-0.10191	0.22690	0.00955		
(SD)	(0.051319)	(0.097619)	(0.119784)	(0.055481)		
Median	0.14000	-0.09290	0.21870	0.01035		
Min, Max	0.0800, 0.2900	-0.3279, 0.0311	0.0383, 0.5375	-0.1279, 0.0948		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	29	29	11	11		
Mean	0.15693	-0.09362	0.13846	-0.04549		
(SD)	(0.054715)	(0.098637)	(0.053174)	(0.076829)		
Median	0.15060	-0.07990	0.14430	-0.04210		
Min, Max	0.0796, 0.3100	-0.3539, 0.0460	0.0291, 0.2132	-0.2200, 0.0841		
Week 14						
n	28	28	12	12		
Mean	0.14755	-0.10408	0.11184	-0.07431		
(SD)	(0.050205)	(0.095582)	(0.048609)	(0.084254)		
Median	0.14770	-0.09560	0.11730	-0.08100		
Min, Max	0.0642, 0.2720	-0.3537, 0.0100	0.0241, 0.2053	-0.2852, 0.0501		
Week 15						
n	26	26	10	10		
Mean	0.14864	-0.08376	0.10519	-0.09131		
(SD)	(0.049911)	(0.099149)	(0.042936)	(0.085851)		
Median	0.14090	-0.07040	0.09585	-0.09915		
Min, Max	0.0736, 0.2517	-0.3686, 0.0437	0.0328, 0.1659	-0.2673, 0.0599		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	27	27	13	13		
Mean	0.15392	-0.08677	0.10777	-0.08462		
(SD)	(0.053183)	(0.096833)	(0.048472)	(0.077998)		
Median	0.14910	-0.05570	0.10000	-0.08930		
Min, Max	0.0819, 0.2986	-0.3234, 0.0749	0.0315, 0.1970	-0.2719, 0.0639		
Week 18						
n	26	26	10	10		
Mean	0.17423	-0.07052	0.12225	-0.06810		
(SD)	(0.064223)	(0.079259)	(0.055963)	(0.075570)		
Median	0.15305	-0.07625	0.11880	-0.07365		
Min, Max	0.0811, 0.3164	-0.2201, 0.0800	0.0461, 0.2076	-0.2256, 0.0396		
Week 20						
n	27	27	10	10		
Mean	0.16261	-0.08853	0.11560	-0.07475		
(SD)	(0.061453)	(0.084066)	(0.050925)	(0.069740)		
Median	0.14590	-0.06700	0.11390	-0.07755		
Min, Max	0.0813, 0.2949	-0.3087, 0.0712	0.0362, 0.2140	-0.2192, 0.0458		

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	27	27	11	11		
Mean	0.15806	-0.08529	0.12467	-0.07266		
(SD)	(0.054007)	(0.105435)	(0.047613)	(0.070439)		
Median	0.14570	-0.06000	0.12710	-0.07560		
Min, Max	0.0708, 0.2932	-0.3880, 0.0695	0.0421, 0.1916	-0.2533, 0.0229		
Week 24						
n	28	28	9	9		
Mean	0.15065	-0.09191	0.15658	-0.06543		
(SD)	(0.060391)	(0.089631)	(0.038090)	(0.088798)		
Median	0.13625	-0.06685	0.15130	-0.05460		
Min, Max	0.0748, 0.3700	-0.3735, 0.0452	0.1016, 0.2273	-0.2671, 0.0346		
Week 28						
n	26	26	9	9	35	35
Mean	0.15483	-0.08394	0.13534	-0.07237	0.14982	-0.08097
(SD)	(0.077178)	(0.067667)	(0.024046)	(0.085966)	(0.067752)	(0.071638)
Median	0.14140	-0.08520	0.13980	-0.05960	0.14080	-0.06830
Min, Max	0.0533, 0.4700	-0.2643, 0.0050	0.1084, 0.1700	-0.2632, 0.0455	0.0533, 0.4700	-0.2643, 0.0455

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	27	27	7	7	34	34
Mean	0.15795	-0.09136	0.13314	-0.08321	0.15284	-0.08968
(SD)	(0.058459)	(0.101223)	(0.039496)	(0.097236)	(0.055497)	(0.099009)
Median	0.15380	-0.05700	0.13070	-0.07280	0.14765	-0.07075
Min, Max	0.0526, 0.2757	-0.3290, 0.0531	0.0689, 0.1900	-0.2627, 0.0289	0.0526, 0.2757	-0.3290, 0.0531
Week 36						
n	21	21	11	11	32	32
Mean	0.15758	-0.09413	0.14174	-0.06189	0.15213	-0.08305
(SD)	(0.062941)	(0.096402)	(0.043417)	(0.067540)	(0.056766)	(0.087802)
Median	0.14380	-0.07850	0.14090	-0.05090	0.14295	-0.06005
Min, Max	0.0750, 0.3384	-0.3076, 0.0860	0.0734, 0.2161	-0.2388, 0.0354	0.0734, 0.3384	-0.3076, 0.0860
Week 40						
n	22	22	7	7	29	29
Mean	0.16383	-0.08181	0.14244	-0.07391	0.15867	-0.07990
(SD)	(0.050227)	(0.090288)	(0.053323)	(0.072004)	(0.050873)	(0.085069)
Median	0.17180	-0.07285	0.14240	-0.07220	0.15810	-0.07220
Min, Max	0.0858, 0.2831	-0.2646, 0.0538	0.0567, 0.2359	-0.1973, 0.0382	0.0567, 0.2831	-0.2646, 0.0538

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

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Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	21	21	8	8	29	29
Mean	0.15972	-0.09059	0.15548	-0.06463	0.15855	-0.08342
(SD)	(0.060622)	(0.078696)	(0.038489)	(0.075693)	(0.054764)	(0.077430)
Median	0.14090	-0.06440	0.15305	-0.06010	0.14090	-0.06440
Min, Max	0.0845, 0.3216	-0.3035, -0.0146	0.1018, 0.2068	-0.2264, 0.0304	0.0845, 0.3216	-0.3035, 0.0304
Week 48						
n	18	18	6	6	24	24
Mean	0.15255	-0.08911	0.14855	-0.07907	0.15155	-0.08660
(SD)	(0.054727)	(0.069433)	(0.039803)	(0.077779)	(0.050609)	(0.069987)
Median	0.12545	-0.09025	0.13460	-0.06470	0.12800	-0.07165
Min, Max	0.0903, 0.2753	-0.2917, 0.0006	0.1148, 0.2241	-0.2091, 0.0281	0.0903, 0.2753	-0.2917, 0.0281
Week 52						
n	14	14	4	4	18	18
Mean	0.14205	-0.08404	0.19328	-0.05288	0.15343	-0.07712
(SD)	(0.036095)	(0.055808)	(0.082972)	(0.059455)	(0.051879)	(0.056421)
Median	0.13370	-0.09200	0.17380	-0.04980	0.14080	-0.08225
Min, Max	0.0871, 0.2081	-0.2135, -0.0200	0.1170, 0.3085	-0.1247, 0.0128	0.0871, 0.3085	-0.2135, 0.0128

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Post-hoc Analysen Studie ALPHA

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Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	20	20	4	4	24	24
Mean	0.14508	-0.09727	0.16895	-0.09658	0.14906	-0.09715
(SD)	(0.059214)	(0.080080)	(0.068161)	(0.056454)	(0.059875)	(0.075586)
Median	0.12820	-0.09140	0.14040	-0.09045	0.12980	-0.09140
Min, Max	0.0439, 0.2581	-0.3276, -0.0100	0.1245, 0.2705	-0.1627, -0.0427	0.0439, 0.2705	-0.3276, -0.0100
Week 60						
n	14	14	6	6	20	20
Mean	0.16464	-0.10371	0.17515	-0.04143	0.16779	-0.08503
(SD)	(0.060207)	(0.079713)	(0.068780)	(0.065852)	(0.061234)	(0.079663)
Median	0.14735	-0.08680	0.15600	-0.04215	0.15055	-0.07180
Min, Max	0.0906, 0.2700	-0.2899, -0.0191	0.1246, 0.3121	-0.1211, 0.0550	0.0906, 0.3121	-0.2899, 0.0550
Week 64						
n	13	13	5	5	18	18
Mean	0.14305	-0.10719	0.17162	-0.06164	0.15099	-0.09454
(SD)	(0.079216)	(0.071137)	(0.071614)	(0.063506)	(0.076221)	(0.070440)
Median	0.12570	-0.11640	0.16270	-0.07240	0.13155	-0.09795
Min, Max	0.0500, 0.3500	-0.2592, -0.0110	0.1093, 0.2906	-0.1426, 0.0185	0.0500, 0.3500	-0.2592, 0.0185

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Post-hoc Analysen Studie ALPHA

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Table GERMANY.2.6.4.7

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Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	9	9	4	4	13	13
Mean	0.12480	-0.10749	0.13958	-0.03158	0.12935	-0.08413
(SD)	(0.056124)	(0.037168)	(0.046129)	(0.038984)	(0.051791)	(0.051292)
Median	0.11210	-0.10820	0.13820	-0.03775	0.12480	-0.09960
Min, Max	0.0637, 0.2565	-0.1565, -0.0445	0.0861, 0.1958	-0.0714, 0.0206	0.0637, 0.2565	-0.1565, 0.0206
Week 72						
n	10	10	3	3	13	13
Mean	0.18629	-0.08395	0.22697	-0.01217	0.19568	-0.06738
(SD)	(0.099104)	(0.065735)	(0.115558)	(0.058891)	(0.099549)	(0.069352)
Median	0.14050	-0.07105	0.17330	-0.00670	0.15100	-0.07000
Min, Max	0.0932, 0.3833	-0.2295, -0.0002	0.1480, 0.3596	-0.0736, 0.0438	0.0932, 0.3833	-0.2295, 0.0438
Week 80						
n	7	7	3	3	10	10
Mean	0.16957	-0.08463	0.13417	-0.10497	0.15895	-0.09073
(SD)	(0.091746)	(0.065845)	(0.012535)	(0.169376)	(0.077065)	(0.096758)
Median	0.13330	-0.09640	0.14100	-0.06030	0.13715	-0.07835
Min, Max	0.0829, 0.3500	-0.1795, 0.0200	0.1197, 0.1418	-0.2922, 0.0376	0.0829, 0.3500	-0.2922, 0.0376

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB
Run Date: 2024-04-26T15:53:53

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.7

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	3	3	1	1	4	4
Mean	0.13597	-0.17257	0.28090	-0.15230	0.17220	-0.16750
(SD)	(0.071042)	(0.163595)	(NA)	(NA)	(0.092823)	(0.133959)
Median	0.11360	-0.08700	0.28090	-0.15230	0.16455	-0.11965
Min, Max	0.0788, 0.2155	-0.3612, -0.0695	0.2809, 0.2809	-0.1523, -0.1523	0.0788, 0.2809	-0.3612, -0.0695
Week 96						
n	2	2	1	1	3	3
Mean	0.18010	-0.06065	0.10870	-0.32450	0.15630	-0.14860
(SD)	(0.013152)	(0.049427)	(NA)	(NA)	(0.042259)	(0.156292)
Median	0.18010	-0.06065	0.10870	-0.32450	0.17080	-0.09560
Min, Max	0.1708, 0.1894	-0.0956, -0.0257	0.1087, 0.1087	-0.3245, -0.3245	0.1087, 0.1894	-0.3245, -0.0257
Week 104						
n	1	1	1	1	2	2
Mean	0.17020	-0.11480	0.07600	-0.05720	0.12310	-0.08600
(SD)	(NA)	(NA)	(NA)	(NA)	(0.066609)	(0.040729)
Median	0.17020	-0.11480	0.07600	-0.05720	0.12310	-0.08600
Min, Max	0.1702, 0.1702	-0.1148, -0.1148	0.0760, 0.0760	-0.0572, -0.0572	0.0760, 0.1702	-0.1148, -0.0572

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean	0.22416		0.18790		0.21208	
(SD)	(0.075738)		(0.030778)		(0.065019)	
Median	0.19715		0.19055		0.19705	
Min, Max	0.1544, 0.3763		0.1488, 0.2217		0.1488, 0.3763	
Week 1						
n	7	7	4	4		
Mean	0.17257	-0.06004	0.19378	0.00588		
(SD)	(0.049773)	(0.060362)	(0.062333)	(0.037175)		
Median	0.16910	-0.07800	0.21770	0.01570		
Min, Max	0.1123, 0.2296	-0.1641, 0.0183	0.1015, 0.2382	-0.0473, 0.0394		
Week 2						
n	7	7	3	3		
Mean	0.13040	-0.10221	0.19530	0.01867		
(SD)	(0.053783)	(0.082989)	(0.061769)	(0.042519)		
Median	0.13310	-0.10010	0.21660	0.01720		
Min, Max	0.0419, 0.2009	-0.2566, -0.0213	0.1257, 0.2436	-0.0231, 0.0619		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	7	7	3	3		
Mean	0.12716	-0.10546	0.18990	0.01327		
(SD)	(0.045946)	(0.094496)	(0.043992)	(0.027463)		
Median	0.14400	-0.08510	0.20280	0.00340		
Min, Max	0.0564, 0.1858	-0.2957, -0.0104	0.1409, 0.2260	-0.0079, 0.0443		
Week 4						
n	7	7	3	3		
Mean	0.14517	-0.08744	0.19247	0.01583		
(SD)	(0.055285)	(0.105090)	(0.049200)	(0.027285)		
Median	0.16700	-0.02770	0.21890	0.01950		
Min, Max	0.0529, 0.1924	-0.2923, -0.0067	0.1357, 0.2228	-0.0131, 0.0411		
Week 6						
n	8	8	3	3		
Mean	0.14289	-0.08128	0.16573	-0.01090		
(SD)	(0.043374)	(0.084997)	(0.055882)	(0.030800)		
Median	0.14895	-0.04075	0.18670	0.00500		
Min, Max	0.0660, 0.1998	-0.2686, -0.0146	0.1024, 0.2081	-0.0464, 0.0087		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	7	7	4	4		
Mean	0.15881	-0.05437	0.20468	0.01678		
(SD)	(0.042928)	(0.085220)	(0.044242)	(0.022482)		
Median	0.15710	-0.00040	0.20910	0.01500		
Min, Max	0.0894, 0.2109	-0.2192, 0.0106	0.1482, 0.2523	-0.0040, 0.0411		
Week 10						
n	6	6	4	4		
Mean	0.17382	-0.06235	0.19480	0.00690		
(SD)	(0.034547)	(0.062666)	(0.047170)	(0.031496)		
Median	0.16515	-0.05050	0.18945	0.01490		
Min, Max	0.1362, 0.2299	-0.1783, 0.0068	0.1485, 0.2518	-0.0347, 0.0325		
Week 12						
n	8	8	4	4		
Mean	0.16329	-0.06088	0.21745	0.02955		
(SD)	(0.056094)	(0.098194)	(0.037032)	(0.029203)		
Median	0.16590	-0.02515	0.22395	0.01765		
Min, Max	0.0846, 0.2630	-0.2917, 0.0085	0.1672, 0.2547	0.0099, 0.0730		

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	8	8	4	4		
Mean	0.17030	-0.05386	0.13120	-0.05670		
(SD)	(0.057335)	(0.101043)	(0.035130)	(0.031984)		
Median	0.16645	-0.01510	0.12400	-0.05520		
Min, Max	0.0893, 0.2456	-0.2765, 0.0460	0.0966, 0.1802	-0.0972, -0.0192		
Week 14						
n	8	8	4	4		
Mean	0.15403	-0.07014	0.11493	-0.07298		
(SD)	(0.049011)	(0.088719)	(0.062540)	(0.038008)		
Median	0.16280	-0.03115	0.09540	-0.09000		
Min, Max	0.0642, 0.2158	-0.2621, -0.0012	0.0636, 0.2053	-0.0955, -0.0164		
Week 15						
n	8	8	3	3		
Mean	0.16528	-0.05889	0.08933	-0.11160		
(SD)	(0.041167)	(0.096488)	(0.022621)	(0.026550)		
Median	0.17645	-0.01100	0.08350	-0.11150		
Min, Max	0.0985, 0.2132	-0.2654, 0.0189	0.0702, 0.1143	-0.1382, -0.0851		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	8	8	4	4		
Mean	0.16154	-0.06263	0.08010	-0.10780		
(SD)	(0.044918)	(0.101628)	(0.022524)	(0.020377)		
Median	0.17440	-0.01570	0.08120	-0.10025		
Min, Max	0.0952, 0.2236	-0.2811, 0.0240	0.0516, 0.1064	-0.1377, -0.0930		
Week 18						
n	8	8	4	4		
Mean	0.17355	-0.05061	0.11418	-0.07373		
(SD)	(0.040864)	(0.081408)	(0.035175)	(0.054727)		
Median	0.16670	-0.02980	0.11650	-0.09405		
Min, Max	0.1174, 0.2428	-0.2201, 0.0432	0.0689, 0.1548	-0.1128, 0.0060		
Week 20						
n	8	8	4	4		
Mean	0.16866	-0.05550	0.10675	-0.08115		
(SD)	(0.051848)	(0.079536)	(0.012532)	(0.025725)		
Median	0.16505	-0.03810	0.10480	-0.08185		
Min, Max	0.0838, 0.2415	-0.2160, 0.0419	0.0946, 0.1228	-0.1115, -0.0494		

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	8	8	4	4		
Mean	0.17289	-0.05128	0.11230	-0.07560		
(SD)	(0.047878)	(0.086676)	(0.035426)	(0.018816)		
Median	0.17405	-0.02690	0.11290	-0.08205		
Min, Max	0.0958, 0.2451	-0.2309, 0.0455	0.0729, 0.1505	-0.0894, -0.0489		
Week 24						
n	7	7	4	4		
Mean	0.15876	-0.06891	0.13430	-0.05360		
(SD)	(0.053944)	(0.094029)	(0.032488)	(0.057097)		
Median	0.13590	-0.05880	0.13035	-0.06735		
Min, Max	0.0748, 0.2173	-0.2446, 0.0452	0.1016, 0.1749	-0.1058, 0.0261		
Week 28						
n	6	6	4	4	10	10
Mean	0.14060	-0.08980	0.11753	-0.07038	0.13137	-0.08203
(SD)	(0.038893)	(0.096853)	(0.015032)	(0.030838)	(0.032522)	(0.075026)
Median	0.14385	-0.07925	0.11095	-0.06395	0.12660	-0.06395
Min, Max	0.0843, 0.1952	-0.2643, -0.0006	0.1084, 0.1398	-0.1132, -0.0404	0.0843, 0.1952	-0.2643, -0.0006

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	8	8	3	3	11	11
Mean	0.17223	-0.05194	0.10283	-0.07380	0.15330	-0.05790
(SD)	(0.062638)	(0.089627)	(0.031343)	(0.005667)	(0.063195)	(0.075722)
Median	0.15760	-0.02930	0.10890	-0.07280	0.14210	-0.03660
Min, Max	0.0882, 0.2757	-0.2471, 0.0531	0.0689, 0.1307	-0.0799, -0.0687	0.0689, 0.2757	-0.2471, 0.0531
Week 36						
n	7	7	3	3	10	10
Mean	0.15783	-0.06984	0.11503	-0.06160	0.14499	-0.06737
(SD)	(0.054436)	(0.077857)	(0.036407)	(0.012541)	(0.051937)	(0.063968)
Median	0.15240	-0.04470	0.13080	-0.05850	0.14150	-0.05175
Min, Max	0.0750, 0.2563	-0.2239, 0.0091	0.0734, 0.1409	-0.0754, -0.0509	0.0734, 0.2563	-0.2239, 0.0091
Week 40						
n	6	6	3	3	9	9
Mean	0.15820	-0.07992	0.10287	-0.07377	0.13976	-0.07787
(SD)	(0.052305)	(0.072082)	(0.040001)	(0.017602)	(0.053622)	(0.057744)
Median	0.15370	-0.07490	0.12470	-0.07220	0.12720	-0.07470
Min, Max	0.0898, 0.2263	-0.2062, -0.0052	0.0567, 0.1272	-0.0921, -0.0570	0.0567, 0.2263	-0.2062, -0.0052

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	5	5	2	2	7	7
Mean	0.16344	-0.04704	0.12550	-0.06505	0.15260	-0.05219
(SD)	(0.068355)	(0.031457)	(0.002263)	(0.014779)	(0.058810)	(0.027809)
Median	0.13840	-0.02970	0.12550	-0.06505	0.13140	-0.05460
Min, Max	0.0927, 0.2713	-0.0983, -0.0230	0.1239, 0.1271	-0.0755, -0.0546	0.0927, 0.2713	-0.0983, -0.0230
Week 48						
n	4	4	2	2	6	6
Mean	0.17395	-0.04140	0.12585	-0.06470	0.15792	-0.04917
(SD)	(0.064290)	(0.026410)	(0.015627)	(0.003111)	(0.056087)	(0.023774)
Median	0.16115	-0.03675	0.12585	-0.06470	0.13180	-0.05415
Min, Max	0.1183, 0.2552	-0.0764, -0.0157	0.1148, 0.1369	-0.0669, -0.0625	0.1148, 0.2552	-0.0764, -0.0157
Week 52						
n	4	4	0	0	4	4
Mean	0.14370	-0.07165			0.14370	-0.07165
(SD)	(0.055837)	(0.033652)			(0.055837)	(0.033652)
Median	0.13980	-0.06945			0.13980	-0.06945
Min, Max	0.0871, 0.2081	-0.1076, -0.0401			0.0871, 0.2081	-0.1076, -0.0401

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	4	4	1	1	5	5
Mean	0.16493	-0.05043	0.12450	-0.05720	0.15684	-0.05178
(SD)	(0.074438)	(0.037686)	(NA)	(NA)	(0.066952)	(0.032777)
Median	0.15625	-0.03650	0.12450	-0.05720	0.12450	-0.04290
Min, Max	0.0891, 0.2581	-0.1056, -0.0231	0.1245, 0.1245	-0.0572, -0.0572	0.0891, 0.2581	-0.1056, -0.0231
Week 60						
n	4	4	1	1	5	5
Mean	0.17060	-0.04475	0.12460	-0.05710	0.16140	-0.04722
(SD)	(0.070828)	(0.044865)	(NA)	(NA)	(0.064696)	(0.039245)
Median	0.17205	-0.03620	0.12460	-0.05710	0.13530	-0.05330
Min, Max	0.0906, 0.2477	-0.1041, -0.0025	0.1246, 0.1246	-0.0571, -0.0571	0.0906, 0.2477	-0.1041, -0.0025
Week 64						
n	3	3	1	1	4	4
Mean	0.14170	-0.04510	0.10930	-0.07240	0.13360	-0.05193
(SD)	(0.062567)	(0.061767)	(NA)	(NA)	(0.053593)	(0.052247)
Median	0.14340	-0.01100	0.10930	-0.07240	0.12635	-0.04170
Min, Max	0.0783, 0.2034	-0.1164, -0.0079	0.1093, 0.1093	-0.0724, -0.0724	0.0783, 0.2034	-0.1164, -0.0079

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	3	3	0	0	3	3
Mean	0.17297	-0.06270			0.17297	-0.06270
(SD)	(0.081301)	(0.032831)			(0.081301)	(0.032831)
Median	0.16830	-0.04450			0.16830	-0.04450
Min, Max	0.0941, 0.2565	-0.1006, -0.0430			0.0941, 0.2565	-0.1006, -0.0430
Week 72						
n	1	1	0	0	1	1
Mean	0.09320	-0.10150			0.09320	-0.10150
(SD)	(NA)	(NA)			(NA)	(NA)
Median	0.09320	-0.10150			0.09320	-0.10150
Min, Max	0.0932, 0.0932	-0.1015, -0.1015			0.0932, 0.0932	-0.1015, -0.1015

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

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Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	49		21		70	
Mean	0.25145		0.22470		0.24342	
(SD)	(0.100380)		(0.128252)		(0.109223)	
Median	0.22390		0.18000		0.22065	
Min, Max	0.1094, 0.5295		0.0394, 0.5419		0.0394, 0.5419	
Week 1						
n	40	40	17	16		
Mean	0.16676	-0.08088	0.21114	0.00437		
(SD)	(0.062368)	(0.064438)	(0.101781)	(0.043102)		
Median	0.15815	-0.08130	0.21680	0.00270		
Min, Max	0.0553, 0.3200	-0.2457, 0.0100	0.0410, 0.4444	-0.0975, 0.0929		
Week 2						
n	46	46	19	18		
Mean	0.12279	-0.13092	0.22494	0.00071		
(SD)	(0.056768)	(0.101951)	(0.104951)	(0.031023)		
Median	0.11255	-0.09940	0.20630	-0.00400		
Min, Max	0.0388, 0.3474	-0.4371, 0.0089	0.0402, 0.4748	-0.0671, 0.0686		

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	39	39	17	17		
Mean	0.11332	-0.12699	0.21360	-0.00706		
(SD)	(0.058638)	(0.093469)	(0.110541)	(0.038547)		
Median	0.10000	-0.11050	0.20830	-0.00850		
Min, Max	0.0400, 0.2506	-0.4571, 0.0148	0.0309, 0.5193	-0.1185, 0.0529		
Week 4						
n	43	43	18	18		
Mean	0.11909	-0.12280	0.21055	-0.01359		
(SD)	(0.055057)	(0.093439)	(0.123883)	(0.047761)		
Median	0.11000	-0.11950	0.17610	-0.00030		
Min, Max	0.0326, 0.2846	-0.3445, 0.1083	0.0388, 0.5743	-0.1138, 0.0579		
Week 6						
n	39	39	19	19		
Mean	0.12702	-0.10858	0.23138	0.00194		
(SD)	(0.053280)	(0.073771)	(0.122656)	(0.034304)		
Median	0.11200	-0.10110	0.22020	0.00430		
Min, Max	0.0554, 0.3100	-0.3016, 0.0255	0.0343, 0.5254	-0.0716, 0.0611		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	37	37	18	18		
Mean	0.13338	-0.10186	0.21069	-0.00493		
(SD)	(0.050688)	(0.071720)	(0.099410)	(0.047049)		
Median	0.12000	-0.09870	0.20375	-0.00800		
Min, Max	0.0682, 0.3244	-0.2995, 0.0651	0.0424, 0.4560	-0.0859, 0.0827		
Week 10						
n	31	31	16	16		
Mean	0.15066	-0.10272	0.22909	0.00896		
(SD)	(0.057945)	(0.078349)	(0.110572)	(0.051874)		
Median	0.13430	-0.08990	0.19670	0.01190		
Min, Max	0.0534, 0.3238	-0.2859, 0.0645	0.1180, 0.5817	-0.1417, 0.0855		
Week 12						
n	37	37	18	18		
Mean	0.15147	-0.09882	0.22984	-0.00346		
(SD)	(0.058447)	(0.093901)	(0.125613)	(0.051972)		
Median	0.14000	-0.09170	0.21430	-0.00520		
Min, Max	0.0612, 0.3537	-0.3279, 0.0944	0.0383, 0.5375	-0.1279, 0.0948		

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	30	30	16	15		
Mean	0.14648	-0.09897	0.17142	-0.03407		
(SD)	(0.052335)	(0.089412)	(0.062633)	(0.083998)		
Median	0.14495	-0.08015	0.17550	-0.02220		
Min, Max	0.0495, 0.3100	-0.3539, 0.0076	0.0291, 0.3126	-0.2200, 0.0841		
Week 14						
n	32	32	18	18		
Mean	0.14734	-0.10447	0.12472	-0.08416		
(SD)	(0.045960)	(0.088190)	(0.037642)	(0.109021)		
Median	0.14185	-0.09645	0.13005	-0.06760		
Min, Max	0.0733, 0.2720	-0.3537, 0.0100	0.0241, 0.1988	-0.3603, 0.0501		
Week 15						
n	31	31	16	15		
Mean	0.14372	-0.08950	0.12344	-0.08227		
(SD)	(0.047779)	(0.087789)	(0.043049)	(0.084628)		
Median	0.12820	-0.08610	0.11970	-0.05740		
Min, Max	0.0736, 0.2517	-0.3686, 0.0437	0.0328, 0.2208	-0.2673, 0.0599		

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	34	34	19	18		
Mean	0.15278	-0.08752	0.12901	-0.08506		
(SD)	(0.048396)	(0.081554)	(0.057666)	(0.085019)		
Median	0.15060	-0.06340	0.12100	-0.07050		
Min, Max	0.0819, 0.2986	-0.3234, 0.0749	0.0315, 0.3029	-0.2719, 0.0639		
Week 18						
n	31	31	14	13		
Mean	0.16795	-0.07080	0.13581	-0.07717		
(SD)	(0.065537)	(0.073953)	(0.061279)	(0.082470)		
Median	0.16380	-0.07890	0.12690	-0.05950		
Min, Max	0.0761, 0.3164	-0.2148, 0.0800	0.0461, 0.2377	-0.2256, 0.0396		
Week 20						
n	33	33	13	12		
Mean	0.15095	-0.09975	0.14597	-0.08248		
(SD)	(0.058331)	(0.078208)	(0.083268)	(0.070590)		
Median	0.14170	-0.08820	0.12440	-0.07045		
Min, Max	0.0538, 0.2949	-0.3087, 0.0712	0.0362, 0.3278	-0.2192, 0.0458		

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	32	32	13	12		
Mean	0.15526	-0.08775	0.14362	-0.06851		
(SD)	(0.055879)	(0.091394)	(0.058459)	(0.081131)		
Median	0.14635	-0.08680	0.12710	-0.05925		
Min, Max	0.0708, 0.2932	-0.3880, 0.0695	0.0421, 0.2625	-0.2533, 0.0531		
Week 24						
n	35	35	10	10		
Mean	0.14598	-0.08641	0.17917	-0.08192		
(SD)	(0.062152)	(0.075596)	(0.051066)	(0.121190)		
Median	0.13660	-0.06830	0.16590	-0.05665		
Min, Max	0.0351, 0.3700	-0.3735, 0.0011	0.1285, 0.2996	-0.2964, 0.0902		
Week 28						
n	33	33	10	9	43	42
Mean	0.15256	-0.08055	0.14344	-0.08008	0.15044	-0.08045
(SD)	(0.069591)	(0.057974)	(0.028299)	(0.087175)	(0.062262)	(0.064079)
Median	0.13540	-0.08090	0.14965	-0.07630	0.14080	-0.07860
Min, Max	0.0533, 0.4700	-0.2564, 0.0050	0.0968, 0.1874	-0.2632, 0.0455	0.0533, 0.4700	-0.2632, 0.0455

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB
Run Date: 2024-04-26T15:53:56

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	32	32	11	10	43	42
Mean	0.15709	-0.08548	0.14918	-0.07026	0.15507	-0.08185
(SD)	(0.055728)	(0.088726)	(0.057379)	(0.103103)	(0.055573)	(0.091262)
Median	0.15155	-0.05965	0.13310	-0.04905	0.14960	-0.05755
Min, Max	0.0526, 0.2700	-0.3290, 0.0260	0.0900, 0.2940	-0.2627, 0.0846	0.0526, 0.2940	-0.3290, 0.0846
Week 36						
n	26	26	13	13	39	39
Mean	0.15638	-0.09560	0.15284	-0.08086	0.15520	-0.09069
(SD)	(0.056092)	(0.084603)	(0.051701)	(0.077342)	(0.054008)	(0.081533)
Median	0.15025	-0.08465	0.14870	-0.06160	0.15000	-0.07540
Min, Max	0.0701, 0.3384	-0.3076, 0.0860	0.0928, 0.2678	-0.2388, 0.0354	0.0701, 0.3384	-0.3076, 0.0860
Week 40						
n	29	29	8	8	37	37
Mean	0.16270	-0.07790	0.18755	-0.07990	0.16808	-0.07834
(SD)	(0.047495)	(0.082648)	(0.094962)	(0.073066)	(0.060129)	(0.079697)
Median	0.15810	-0.05490	0.15210	-0.08065	0.15640	-0.07100
Min, Max	0.0839, 0.2831	-0.2646, 0.0538	0.1109, 0.4052	-0.1973, 0.0382	0.0839, 0.4052	-0.2646, 0.0538

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	29	29	12	12	41	41
Mean	0.16041	-0.09154	0.18213	-0.06104	0.16677	-0.08261
(SD)	(0.059349)	(0.078305)	(0.063067)	(0.089334)	(0.060494)	(0.081756)
Median	0.15460	-0.06660	0.17665	-0.05585	0.16100	-0.06560
Min, Max	0.0675, 0.3216	-0.3035, 0.0464	0.1018, 0.3040	-0.2264, 0.0721	0.0675, 0.3216	-0.3035, 0.0721
Week 48						
n	25	25	10	9	35	34
Mean	0.16264	-0.08625	0.15443	-0.11561	0.16029	-0.09402
(SD)	(0.049418)	(0.068341)	(0.057052)	(0.096520)	(0.050986)	(0.076341)
Median	0.16200	-0.08630	0.14280	-0.10500	0.15330	-0.09670
Min, Max	0.0903, 0.2753	-0.2917, 0.0105	0.0788, 0.2721	-0.3072, 0.0281	0.0788, 0.2753	-0.3072, 0.0281
Week 52						
n	19	19	10	9	29	28
Mean	0.15522	-0.07714	0.13876	-0.07862	0.14954	-0.07762
(SD)	(0.042507)	(0.069545)	(0.069430)	(0.050686)	(0.052673)	(0.063135)
Median	0.14720	-0.06620	0.11625	-0.07340	0.14340	-0.07185
Min, Max	0.0764, 0.2693	-0.2386, -0.0029	0.0600, 0.3085	-0.1428, 0.0128	0.0600, 0.3085	-0.2386, 0.0128

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	25	25	9	8	34	33
Mean	0.15654	-0.09642	0.15903	-0.11238	0.15720	-0.10029
(SD)	(0.056300)	(0.082594)	(0.056793)	(0.073601)	(0.055573)	(0.079684)
Median	0.14000	-0.09100	0.14350	-0.10080	0.14175	-0.09100
Min, Max	0.0439, 0.2662	-0.3276, -0.0079	0.0800, 0.2705	-0.2573, -0.0427	0.0439, 0.2705	-0.3276, -0.0079
Week 60						
n	18	18	10	10	28	28
Mean	0.16594	-0.10283	0.15522	-0.05455	0.16211	-0.08559
(SD)	(0.051343)	(0.081201)	(0.059986)	(0.060972)	(0.053727)	(0.077108)
Median	0.15580	-0.08680	0.14460	-0.05515	0.15220	-0.06890
Min, Max	0.0908, 0.2700	-0.2899, -0.0218	0.1079, 0.3121	-0.1504, 0.0550	0.0908, 0.3121	-0.2899, 0.0550
Week 64						
n	16	16	10	10	26	26
Mean	0.15692	-0.10765	0.14763	-0.08193	0.15335	-0.09776
(SD)	(0.083227)	(0.083839)	(0.064159)	(0.089004)	(0.075227)	(0.085041)
Median	0.13165	-0.10325	0.14670	-0.07115	0.13805	-0.08665
Min, Max	0.0500, 0.3500	-0.2752, 0.0354	0.0500, 0.2906	-0.2974, 0.0185	0.0500, 0.3500	-0.2974, 0.0354

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	12	12	9	9	21	21
Mean	0.14120	-0.10516	0.14049	-0.06889	0.14090	-0.08961
(SD)	(0.058855)	(0.059823)	(0.073587)	(0.053709)	(0.063807)	(0.058826)
Median	0.13575	-0.10390	0.12480	-0.07140	0.13330	-0.08000
Min, Max	0.0637, 0.2867	-0.2357, -0.0030	0.0500, 0.3011	-0.1771, 0.0206	0.0500, 0.3011	-0.2357, 0.0206
Week 72						
n	13	13	8	8	21	21
Mean	0.18389	-0.09145	0.19115	-0.04615	0.18666	-0.07419
(SD)	(0.085469)	(0.079339)	(0.102715)	(0.060015)	(0.089937)	(0.074468)
Median	0.15100	-0.07210	0.14315	-0.05615	0.14980	-0.07000
Min, Max	0.1078, 0.3833	-0.2697, -0.0002	0.1032, 0.3596	-0.1290, 0.0438	0.1032, 0.3833	-0.2697, 0.0438
Week 80						
n	11	11	7	7	18	18
Mean	0.16843	-0.09076	0.12474	-0.12173	0.15144	-0.10281
(SD)	(0.077230)	(0.077660)	(0.036820)	(0.130231)	(0.066837)	(0.098868)
Median	0.13460	-0.09640	0.12840	-0.06070	0.13395	-0.07855
Min, Max	0.0829, 0.3500	-0.2474, 0.0200	0.0505, 0.1697	-0.3085, 0.0376	0.0505, 0.3500	-0.3085, 0.0376

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.8

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	3	3	9	9
Mean	0.15653	-0.11268	0.22537	-0.05853	0.17948	-0.09463
(SD)	(0.051040)	(0.128702)	(0.048097)	(0.088367)	(0.058232)	(0.114183)
Median	0.16970	-0.07825	0.19820	-0.04650	0.19190	-0.06950
Min, Max	0.0788, 0.2155	-0.3612, 0.0073	0.1970, 0.2809	-0.1523, 0.0232	0.0788, 0.2809	-0.3612, 0.0232
Week 96						
n	4	4	4	4	8	8
Mean	0.16990	-0.06693	0.11378	-0.12695	0.14184	-0.09694
(SD)	(0.017241)	(0.041603)	(0.032778)	(0.136454)	(0.038573)	(0.098748)
Median	0.17140	-0.06640	0.12110	-0.07635	0.14530	-0.06855
Min, Max	0.1474, 0.1894	-0.1092, -0.0257	0.0697, 0.1432	-0.3245, -0.0306	0.0697, 0.1894	-0.3245, -0.0257
Week 104						
n	1	1	2	2	3	3
Mean	0.17020	-0.11480	0.13280	-0.05615	0.14527	-0.07570
(SD)	(NA)	(NA)	(0.080327)	(0.001485)	(0.060766)	(0.033878)
Median	0.17020	-0.11480	0.13280	-0.05615	0.17020	-0.05720
Min, Max	0.1702, 0.1702	-0.1148, -0.1148	0.0760, 0.1896	-0.0572, -0.0551	0.0760, 0.1896	-0.1148, -0.0551

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.4.9

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean	0.24025		0.20581		0.22949	
(SD)	(0.099562)		(0.133566)		(0.110260)	
Median	0.20475		0.18085		0.19795	
Min, Max	0.1200, 0.5118		0.0394, 0.5419		0.0394, 0.5419	
Week 1						
n	19	19	10	10		
Mean	0.16682	-0.07315	0.20189	-0.00392		
(SD)	(0.063626)	(0.065485)	(0.111945)	(0.052463)		
Median	0.15080	-0.07870	0.21720	0.00270		
Min, Max	0.0789, 0.2864	-0.2457, 0.0183	0.0410, 0.4444	-0.0975, 0.0929		
Week 2						
n	20	20	9	9		
Mean	0.12975	-0.11325	0.20508	0.00103		
(SD)	(0.066425)	(0.091839)	(0.121574)	(0.036160)		
Median	0.12905	-0.09040	0.20630	0.00080		
Min, Max	0.0419, 0.3474	-0.3658, -0.0213	0.0402, 0.4748	-0.0671, 0.0619		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.6.4.9

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	18	18	9	9		
Mean	0.11395	-0.10639	0.20298	-0.00107		
(SD)	(0.053255)	(0.067150)	(0.133552)	(0.028957)		
Median	0.10785	-0.08985	0.20280	-0.00790		
Min, Max	0.0480, 0.2506	-0.2957, -0.0104	0.0309, 0.5193	-0.0478, 0.0443		
Week 4						
n	19	19	9	9		
Mean	0.11723	-0.12191	0.20320	-0.00084		
(SD)	(0.048637)	(0.101651)	(0.150463)	(0.042778)		
Median	0.10510	-0.11800	0.16790	0.01660		
Min, Max	0.0529, 0.1924	-0.3445, -0.0067	0.0388, 0.5743	-0.0993, 0.0411		
Week 6						
n	22	22	9	9		
Mean	0.13147	-0.10879	0.20254	-0.00150		
(SD)	(0.044712)	(0.089844)	(0.140259)	(0.028984)		
Median	0.12570	-0.08230	0.18670	-0.00410		
Min, Max	0.0554, 0.2257	-0.3016, -0.0146	0.0343, 0.5254	-0.0464, 0.0611		

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Source: ADaM.ADSL, ADaM.ADLB

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Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.9

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	19	19	10	10		
Mean	0.14458	-0.08662	0.20967	0.00386		
(SD)	(0.043167)	(0.084294)	(0.109582)	(0.045211)		
Median	0.15560	-0.07240	0.20910	0.00225		
Min, Max	0.0682, 0.2184	-0.2934, 0.0106	0.0424, 0.4560	-0.0859, 0.0827		
Week 10						
n	17	17	8	8		
Mean	0.15899	-0.10415	0.23978	0.02084		
(SD)	(0.041180)	(0.077196)	(0.146876)	(0.034431)		
Median	0.14900	-0.07890	0.19545	0.02195		
Min, Max	0.0785, 0.2299	-0.2859, 0.0068	0.1180, 0.5817	-0.0347, 0.0826		
Week 12						
n	21	21	10	10		
Mean	0.15574	-0.08529	0.22023	0.01442		
(SD)	(0.045695)	(0.095132)	(0.131953)	(0.042928)		
Median	0.15570	-0.05450	0.21190	0.00615		
Min, Max	0.0846, 0.2630	-0.3279, 0.0311	0.0383, 0.5375	-0.0597, 0.0948		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.9

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	18	18	8	8		
Mean	0.15082	-0.09166	0.12865	-0.02748		
(SD)	(0.044690)	(0.104437)	(0.050198)	(0.052522)		
Median	0.15085	-0.06650	0.13360	-0.03340		
Min, Max	0.0880, 0.2456	-0.3539, 0.0460	0.0291, 0.1883	-0.0972, 0.0841		
Week 14						
n	20	20	8	8		
Mean	0.13911	-0.11094	0.10533	-0.05080		
(SD)	(0.038233)	(0.101262)	(0.054968)	(0.052074)		
Median	0.13585	-0.09645	0.10140	-0.07465		
Min, Max	0.0642, 0.2158	-0.3537, -0.0012	0.0241, 0.2053	-0.0955, 0.0501		
Week 15						
n	20	20	7	7		
Mean	0.14471	-0.10461	0.09636	-0.06081		
(SD)	(0.036008)	(0.109401)	(0.041255)	(0.067510)		
Median	0.14090	-0.09230	0.09320	-0.08510		
Min, Max	0.0891, 0.2132	-0.3686, 0.0189	0.0328, 0.1641	-0.1382, 0.0599		

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.9

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	21	21	9	9		
Mean	0.15104	-0.09494	0.09543	-0.07303		
(SD)	(0.038759)	(0.099712)	(0.041712)	(0.063286)		
Median	0.15330	-0.05920	0.09660	-0.09300		
Min, Max	0.0832, 0.2236	-0.3234, 0.0240	0.0315, 0.1681	-0.1377, 0.0639		
Week 18						
n	18	18	8	8		
Mean	0.16981	-0.09048	0.11069	-0.04544		
(SD)	(0.051565)	(0.080564)	(0.036222)	(0.056610)		
Median	0.16070	-0.08705	0.11775	-0.05745		
Min, Max	0.0916, 0.3164	-0.2201, 0.0432	0.0461, 0.1548	-0.1128, 0.0396		
Week 20						
n	21	21	8	8		
Mean	0.15548	-0.09050	0.10628	-0.04985		
(SD)	(0.048334)	(0.083060)	(0.032939)	(0.049868)		
Median	0.15570	-0.06700	0.11390	-0.05840		
Min, Max	0.0833, 0.2415	-0.3087, 0.0419	0.0362, 0.1500	-0.1115, 0.0458		

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.9

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	18	18	9	9		
Mean	0.14898	-0.09767	0.11668	-0.05179		
(SD)	(0.045147)	(0.109805)	(0.043515)	(0.039559)		
Median	0.14555	-0.08875	0.12040	-0.06030		
Min, Max	0.0708, 0.2451	-0.3880, 0.0455	0.0421, 0.1916	-0.0894, 0.0229		
Week 24						
n	19	19	8	8		
Mean	0.14291	-0.09836	0.14984	-0.03476		
(SD)	(0.040628)	(0.104563)	(0.038340)	(0.047958)		
Median	0.13660	-0.08370	0.14445	-0.03480		
Min, Max	0.0748, 0.2173	-0.3735, 0.0452	0.1016, 0.2273	-0.1058, 0.0346		
Week 28						
n	17	17	8	8	25	25
Mean	0.13648	-0.10342	0.13184	-0.05276	0.13500	-0.08721
(SD)	(0.034385)	(0.079341)	(0.021120)	(0.049271)	(0.030384)	(0.074070)
Median	0.14360	-0.09790	0.13150	-0.05510	0.14200	-0.08320
Min, Max	0.0533, 0.1952	-0.2643, -0.0006	0.1084, 0.1621	-0.1132, 0.0455	0.0533, 0.1952	-0.2643, 0.0455

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.9

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	20	20	7	7	27	27
Mean	0.16405	-0.08304	0.12859	-0.05071	0.15486	-0.07466
(SD)	(0.053183)	(0.095711)	(0.036167)	(0.056900)	(0.051182)	(0.087463)
Median	0.15870	-0.04865	0.13070	-0.06870	0.15320	-0.05170
Min, Max	0.0526, 0.2757	-0.3290, 0.0531	0.0689, 0.1900	-0.1373, 0.0289	0.0526, 0.2757	-0.3290, 0.0531
Week 36						
n	18	18	7	7	25	25
Mean	0.14778	-0.09606	0.13451	-0.04479	0.14406	-0.08170
(SD)	(0.041767)	(0.084641)	(0.030815)	(0.041840)	(0.038859)	(0.077873)
Median	0.14715	-0.06870	0.13960	-0.05090	0.14210	-0.05850
Min, Max	0.0750, 0.2563	-0.3076, 0.0091	0.0734, 0.1723	-0.0949, 0.0354	0.0734, 0.2563	-0.3076, 0.0354
Week 40						
n	18	18	7	7	25	25
Mean	0.15077	-0.08752	0.12866	-0.05064	0.14458	-0.07719
(SD)	(0.038972)	(0.082588)	(0.034168)	(0.047704)	(0.038346)	(0.075404)
Median	0.14740	-0.07855	0.13940	-0.05700	0.14150	-0.07470
Min, Max	0.0858, 0.2263	-0.2543, 0.0538	0.0567, 0.1624	-0.1048, 0.0382	0.0567, 0.2263	-0.2543, 0.0538

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Post-hoc Analysen Studie ALPHA

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Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	17	17	6	6	23	23
Mean	0.14741	-0.08861	0.14443	-0.03995	0.14663	-0.07592
(SD)	(0.047370)	(0.080642)	(0.025407)	(0.043635)	(0.042195)	(0.075097)
Median	0.14090	-0.06440	0.13115	-0.05035	0.13840	-0.05630
Min, Max	0.0845, 0.2713	-0.3035, -0.0146	0.1239, 0.1818	-0.0854, 0.0304	0.0845, 0.2713	-0.3035, 0.0304
Week 48						
n	15	15	6	6	21	21
Mean	0.16737	-0.08991	0.13118	-0.05320	0.15703	-0.07942
(SD)	(0.053297)	(0.078428)	(0.015650)	(0.044628)	(0.048272)	(0.071361)
Median	0.16200	-0.06190	0.12845	-0.05895	0.13690	-0.06190
Min, Max	0.1072, 0.2753	-0.2917, 0.0006	0.1148, 0.1586	-0.1086, 0.0281	0.1072, 0.2753	-0.2917, 0.0281
Week 52						
n	13	13	4	4	17	17
Mean	0.15228	-0.09142	0.14203	-0.03928	0.14987	-0.07915
(SD)	(0.038545)	(0.068910)	(0.040537)	(0.040865)	(0.037981)	(0.066289)
Median	0.14480	-0.09110	0.13540	-0.04825	0.14480	-0.07030
Min, Max	0.0871, 0.2081	-0.2386, -0.0222	0.1035, 0.1938	-0.0734, 0.0128	0.0871, 0.2081	-0.2386, 0.0128

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Post-hoc Analysen Studie ALPHA

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Table GERMANY.2.6.4.9

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	15	15	4	4	19	19
Mean	0.14497	-0.09881	0.13218	-0.06850	0.14227	-0.09243
(SD)	(0.050160)	(0.088546)	(0.009839)	(0.037274)	(0.044741)	(0.080566)
Median	0.12850	-0.08920	0.13090	-0.05380	0.12850	-0.07370
Min, Max	0.0736, 0.2581	-0.3276, -0.0147	0.1234, 0.1435	-0.1237, -0.0427	0.0736, 0.2581	-0.3276, -0.0147
Week 60						
n	13	13	5	5	18	18
Mean	0.15687	-0.10012	0.14362	-0.03776	0.15319	-0.08280
(SD)	(0.048367)	(0.083325)	(0.022137)	(0.058210)	(0.042472)	(0.080774)
Median	0.14640	-0.09630	0.15280	-0.05710	0.14735	-0.05760
Min, Max	0.0906, 0.2477	-0.2899, -0.0025	0.1157, 0.1658	-0.1014, 0.0550	0.0906, 0.2477	-0.2899, 0.0550
Week 64						
n	11	11	5	5	16	16
Mean	0.14428	-0.10505	0.14146	-0.03992	0.14340	-0.08469
(SD)	(0.050926)	(0.090158)	(0.026563)	(0.044673)	(0.043806)	(0.083205)
Median	0.13740	-0.10150	0.13980	-0.03400	0.13860	-0.07730
Min, Max	0.0783, 0.2526	-0.2752, -0.0079	0.1093, 0.1728	-0.0944, 0.0185	0.0783, 0.2526	-0.2752, 0.0185

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Table GERMANY.2.6.4.9

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	9	9	4	4	13	13
Mean	0.14867	-0.09623	0.14705	-0.03425	0.14817	-0.07716
(SD)	(0.058338)	(0.060326)	(0.035854)	(0.040733)	(0.050900)	(0.061053)
Median	0.14630	-0.07450	0.13820	-0.04310	0.14630	-0.06690
Min, Max	0.0637, 0.2565	-0.2357, -0.0430	0.1160, 0.1958	-0.0714, 0.0206	0.0637, 0.2565	-0.2357, 0.0206
Week 72						
n	7	7	3	3	10	10
Mean	0.13576	-0.09783	0.15090	-0.00177	0.14030	-0.06901
(SD)	(0.037419)	(0.080370)	(0.021100)	(0.043311)	(0.032953)	(0.082923)
Median	0.12680	-0.07830	0.14800	-0.00670	0.13970	-0.05825
Min, Max	0.0932, 0.2029	-0.2697, -0.0287	0.1314, 0.1733	-0.0424, 0.0438	0.0932, 0.2029	-0.2697, 0.0438
Week 80						
n	6	6	3	3	9	9
Mean	0.14162	-0.09790	0.12787	-0.02480	0.13703	-0.07353
(SD)	(0.049596)	(0.079023)	(0.012126)	(0.054211)	(0.040267)	(0.077289)
Median	0.12860	-0.07815	0.12210	-0.05170	0.12390	-0.05990
Min, Max	0.1027, 0.2399	-0.2474, -0.0338	0.1197, 0.1418	-0.0603, 0.0376	0.1027, 0.2399	-0.2474, 0.0376

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Table GERMANY.2.6.4.9

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Asian

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	2	2	1	1	3	3
Mean	0.16970	-0.08285	0.19700	0.02320	0.17880	-0.04750
(SD)	(0.008061)	(0.048578)	(NA)	(NA)	(0.016761)	(0.070205)
Median	0.16970	-0.08285	0.19700	0.02320	0.17540	-0.04850
Min, Max	0.1640, 0.1754	-0.1172, -0.0485	0.1970, 0.1970	0.0232, 0.0232	0.1640, 0.1970	-0.1172, 0.0232
Week 96						
n	2	2	1	1	3	3
Mean	0.17140	-0.06745	0.14320	-0.03060	0.16200	-0.05517
(SD)	(0.000849)	(0.059043)	(NA)	(NA)	(0.016292)	(0.046858)
Median	0.17140	-0.06745	0.14320	-0.03060	0.17080	-0.03060
Min, Max	0.1708, 0.1720	-0.1092, -0.0257	0.1432, 0.1432	-0.0306, -0.0306	0.1432, 0.1720	-0.1092, -0.0257

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.9

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		11		39	
Mean	0.23638		0.22935		0.23440	
(SD)	(0.074229)		(0.122754)		(0.088829)	
Median	0.22610		0.20940		0.22100	
Min, Max	0.1094, 0.4400		0.1112, 0.4782		0.1094, 0.4782	
Week 1						
n	23	23	7	7		
Mean	0.16654	-0.07136	0.18154	0.00267		
(SD)	(0.048854)	(0.062131)	(0.049118)	(0.021865)		
Median	0.16090	-0.04950	0.16620	-0.00210		
Min, Max	0.0553, 0.2400	-0.2100, 0.0100	0.1200, 0.2426	-0.0274, 0.0395		
Week 2						
n	26	26	8	8		
Mean	0.11908	-0.12049	0.21004	-0.00063		
(SD)	(0.047925)	(0.090699)	(0.082199)	(0.027164)		
Median	0.11240	-0.09940	0.18525	-0.00535		
Min, Max	0.0388, 0.2299	-0.3609, 0.0089	0.1367, 0.3825	-0.0507, 0.0400		

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White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	21	21	7	7		
Mean	0.11142	-0.11831	0.21581	-0.00637		
(SD)	(0.053133)	(0.086089)	(0.059685)	(0.054137)		
Median	0.10410	-0.12100	0.21740	0.00710		
Min, Max	0.0400, 0.2358	-0.4000, 0.0148	0.1400, 0.3147	-0.1185, 0.0529		
Week 4						
n	25	25	9	9		
Mean	0.12225	-0.10906	0.22551	-0.01488		
(SD)	(0.059286)	(0.092858)	(0.094649)	(0.056166)		
Median	0.11140	-0.11190	0.22480	0.00000		
Min, Max	0.0326, 0.2846	-0.3350, 0.1083	0.1280, 0.3644	-0.1138, 0.0579		
Week 6						
n	19	19	9	9		
Mean	0.11982	-0.09035	0.23781	-0.00258		
(SD)	(0.047055)	(0.058548)	(0.104789)	(0.039908)		
Median	0.11020	-0.10110	0.22020	0.00990		
Min, Max	0.0649, 0.2465	-0.1800, 0.0255	0.1000, 0.4066	-0.0716, 0.0301		

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Table GERMANY.2.6.4.9

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White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	21	21	8	8		
Mean	0.13307	-0.08661	0.21523	0.00456		
(SD)	(0.053269)	(0.053758)	(0.086245)	(0.050286)		
Median	0.12000	-0.09870	0.20375	0.00155		
Min, Max	0.0839, 0.3244	-0.1634, 0.0651	0.1000, 0.3592	-0.0740, 0.0764		
Week 10						
n	15	15	8	8		
Mean	0.15853	-0.06265	0.20981	0.00571		
(SD)	(0.067438)	(0.057389)	(0.050850)	(0.066775)		
Median	0.13340	-0.06950	0.19670	0.01635		
Min, Max	0.0534, 0.3238	-0.1659, 0.0645	0.1400, 0.2915	-0.1417, 0.0855		
Week 12						
n	18	18	9	9		
Mean	0.15228	-0.06897	0.24697	-0.00433		
(SD)	(0.066472)	(0.078807)	(0.108602)	(0.063264)		
Median	0.13745	-0.06985	0.23500	-0.00970		
Min, Max	0.0612, 0.3537	-0.3000, 0.0944	0.1000, 0.4441	-0.1279, 0.0801		

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White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	15	15	7	7		
Mean	0.14389	-0.08291	0.19330	-0.02876		
(SD)	(0.054235)	(0.082889)	(0.029789)	(0.096911)		
Median	0.15060	-0.06670	0.18550	-0.00530		
Min, Max	0.0495, 0.2536	-0.2800, 0.0076	0.1500, 0.2309	-0.2200, 0.0743		
Week 14						
n	15	15	10	10		
Mean	0.14983	-0.08084	0.14116	-0.09613		
(SD)	(0.046801)	(0.063225)	(0.024230)	(0.131737)		
Median	0.16670	-0.06300	0.13160	-0.04540		
Min, Max	0.0804, 0.2066	-0.2400, 0.0100	0.1179, 0.1988	-0.3603, 0.0290		
Week 15						
n	16	16	7	7		
Mean	0.15200	-0.06150	0.13727	-0.10037		
(SD)	(0.054230)	(0.048705)	(0.045239)	(0.089063)		
Median	0.16020	-0.07600	0.12300	-0.11180		
Min, Max	0.0736, 0.2517	-0.1292, 0.0189	0.0900, 0.2208	-0.2673, 0.0114		

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White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	20	20	10	10		
Mean	0.15830	-0.06736	0.15017	-0.08712		
(SD)	(0.056800)	(0.067382)	(0.064396)	(0.088341)		
Median	0.14950	-0.05460	0.13460	-0.05245		
Min, Max	0.0819, 0.2986	-0.2473, 0.0749	0.0891, 0.3029	-0.2719, 0.0193		
Week 18						
n	17	17	6	6		
Mean	0.16978	-0.04485	0.15820	-0.08400		
(SD)	(0.069388)	(0.055857)	(0.060637)	(0.089934)		
Median	0.17040	-0.03260	0.18035	-0.05750		
Min, Max	0.0761, 0.2993	-0.1235, 0.0756	0.0800, 0.2144	-0.2256, 0.0050		
Week 20						
n	16	16	5	5		
Mean	0.15221	-0.07971	0.17848	-0.13112		
(SD)	(0.062061)	(0.075404)	(0.097320)	(0.061992)		
Median	0.14705	-0.07755	0.15650	-0.13060		
Min, Max	0.0538, 0.2949	-0.2686, 0.0712	0.0800, 0.3278	-0.2192, -0.0500		

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White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	17	17	4	4		
Mean	0.16930	-0.06344	0.17890	-0.10840		
(SD)	(0.068141)	(0.074825)	(0.067012)	(0.127745)		
Median	0.16000	-0.07950	0.17730	-0.11670		
Min, Max	0.0727, 0.2932	-0.2434, 0.0695	0.0985, 0.2625	-0.2533, 0.0531		
Week 24						
n	17	17	5	5		
Mean	0.13647	-0.06592	0.19290	-0.13258		
(SD)	(0.057168)	(0.041547)	(0.064018)	(0.156791)		
Median	0.13180	-0.05500	0.18180	-0.11620		
Min, Max	0.0351, 0.2408	-0.1259, 0.0011	0.1285, 0.2996	-0.2964, 0.0902		
Week 28						
n	18	18	2	2	20	20
Mean	0.14847	-0.05944	0.16480	-0.17415	0.15011	-0.07091
(SD)	(0.045505)	(0.038290)	(0.007354)	(0.125936)	(0.043369)	(0.058251)
Median	0.12810	-0.06065	0.16480	-0.17415	0.13630	-0.06630
Min, Max	0.0932, 0.2376	-0.1281, 0.0050	0.1596, 0.1700	-0.2632, -0.0851	0.0932, 0.2376	-0.2632, 0.0050

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Modified Randomized Set

White

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	15	15	4	4	19	19
Mean	0.15402	-0.06089	0.16305	-0.09128	0.15592	-0.06729
(SD)	(0.057751)	(0.077213)	(0.094535)	(0.148383)	(0.064014)	(0.092025)
Median	0.13970	-0.03470	0.13410	-0.09350	0.13970	-0.04000
Min, Max	0.0748, 0.2538	-0.3003, 0.0260	0.0900, 0.2940	-0.2627, 0.0846	0.0748, 0.2940	-0.3003, 0.0846
Week 36						
n	11	11	6	6	17	17
Mean	0.16359	-0.05723	0.18167	-0.10972	0.16997	-0.07575
(SD)	(0.071508)	(0.057192)	(0.059272)	(0.091057)	(0.066129)	(0.072828)
Median	0.15000	-0.07540	0.18125	-0.06910	0.16810	-0.07540
Min, Max	0.0701, 0.3384	-0.1203, 0.0860	0.0949, 0.2678	-0.2388, -0.0251	0.0701, 0.3384	-0.2388, 0.0860
Week 40						
n	13	13	3	3	16	16
Mean	0.16482	-0.06340	0.26583	-0.11953	0.18376	-0.07393
(SD)	(0.048208)	(0.067787)	(0.127072)	(0.067781)	(0.075300)	(0.069287)
Median	0.17540	-0.04090	0.23590	-0.08830	0.17770	-0.06295
Min, Max	0.0839, 0.2358	-0.2646, 0.0100	0.1564, 0.4052	-0.1973, -0.0730	0.0839, 0.4052	-0.2646, 0.0100

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	12	12	6	6	18	18
Mean	0.16944	-0.07086	0.22682	-0.07342	0.18857	-0.07171
(SD)	(0.065904)	(0.067470)	(0.053634)	(0.114854)	(0.066566)	(0.082625)
Median	0.16660	-0.05895	0.20645	-0.07205	0.19815	-0.06270
Min, Max	0.0675, 0.3057	-0.2311, 0.0464	0.1662, 0.3040	-0.2264, 0.0721	0.0675, 0.3057	-0.2311, 0.0721
Week 48						
n	9	9	3	3	12	12
Mean	0.16680	-0.06109	0.16823	-0.21713	0.16716	-0.10010
(SD)	(0.052787)	(0.044831)	(0.057300)	(0.086331)	(0.051224)	(0.088303)
Median	0.16250	-0.07140	0.17100	-0.20910	0.16675	-0.09520
Min, Max	0.0903, 0.2600	-0.1103, 0.0105	0.1096, 0.2241	-0.3072, -0.1351	0.0903, 0.2600	-0.3072, 0.0105
Week 52						
n	7	7	3	3	10	10
Mean	0.15670	-0.04090	0.15983	-0.10947	0.15764	-0.06147
(SD)	(0.061768)	(0.045451)	(0.131250)	(0.034474)	(0.079837)	(0.052329)
Median	0.14820	-0.02120	0.11100	-0.12470	0.14770	-0.04590
Min, Max	0.0764, 0.2693	-0.1151, -0.0029	0.0600, 0.3085	-0.1337, -0.0700	0.0600, 0.3085	-0.1337, -0.0029

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Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:53:58

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.9

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	9	9	4	4	13	13
Mean	0.17322	-0.06441	0.18455	-0.13698	0.17671	-0.08674
(SD)	(0.055612)	(0.043056)	(0.081557)	(0.093442)	(0.061272)	(0.068073)
Median	0.17670	-0.06890	0.19385	-0.12030	0.17670	-0.07790
Min, Max	0.0996, 0.2662	-0.1240, -0.0079	0.0800, 0.2705	-0.2573, -0.0500	0.0800, 0.2705	-0.2573, -0.0079
Week 60						
n	6	6	3	3	9	9
Mean	0.16570	-0.05828	0.19783	-0.07147	0.17641	-0.06268
(SD)	(0.060576)	(0.038075)	(0.103611)	(0.050575)	(0.072355)	(0.039862)
Median	0.15920	-0.04875	0.17140	-0.07330	0.16680	-0.06450
Min, Max	0.0974, 0.2375	-0.1112, -0.0218	0.1100, 0.3121	-0.1211, -0.0200	0.0974, 0.3121	-0.1211, -0.0200
Week 64						
n	5	5	5	5	10	10
Mean	0.16460	-0.04268	0.15350	-0.12596	0.15905	-0.08432
(SD)	(0.093209)	(0.056380)	(0.092161)	(0.105476)	(0.087581)	(0.091016)
Median	0.13870	-0.04590	0.15360	-0.09110	0.14435	-0.07280
Min, Max	0.0833, 0.3251	-0.1173, 0.0354	0.0500, 0.2906	-0.2974, -0.0187	0.0500, 0.3251	-0.2974, 0.0354

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Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.9

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	4	4	3	3	7	7
Mean	0.16390	-0.07965	0.16590	-0.11840	0.16476	-0.09626
(SD)	(0.083640)	(0.064769)	(0.126658)	(0.051635)	(0.094055)	(0.058440)
Median	0.13395	-0.07955	0.14660	-0.09810	0.14280	-0.09810
Min, Max	0.1010, 0.2867	-0.1565, -0.0030	0.0500, 0.3011	-0.1771, -0.0800	0.0500, 0.3011	-0.1771, -0.0030
Week 72						
n	4	4	4	4	8	8
Mean	0.20468	-0.07645	0.24333	-0.07350	0.22400	-0.07498
(SD)	(0.119679)	(0.051158)	(0.128424)	(0.063232)	(0.116763)	(0.053270)
Median	0.15345	-0.06440	0.24375	-0.09000	0.15345	-0.07285
Min, Max	0.1285, 0.3833	-0.1483, -0.0287	0.1262, 0.3596	-0.1290, 0.0150	0.1262, 0.3833	-0.1483, 0.0150
Week 80						
n	3	3	4	4	7	7
Mean	0.14433	-0.08383	0.12240	-0.19443	0.13180	-0.14703
(SD)	(0.053210)	(0.065172)	(0.050954)	(0.124578)	(0.048779)	(0.112561)
Median	0.17420	-0.11770	0.13470	-0.20425	0.14100	-0.11770
Min, Max	0.0829, 0.1759	-0.1251, -0.0087	0.0505, 0.1697	-0.3085, -0.0607	0.0505, 0.1759	-0.3085, -0.0087

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Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.9

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	3	3	2	2	5	5
Mean	0.12810	-0.14697	0.23955	-0.09940	0.17268	-0.12794
(SD)	(0.057927)	(0.191429)	(0.058478)	(0.074812)	(0.079114)	(0.142830)
Median	0.11360	-0.08700	0.23955	-0.09940	0.19190	-0.08700
Min, Max	0.0788, 0.1919	-0.3612, 0.0073	0.1982, 0.2809	-0.1523, -0.0465	0.0788, 0.2809	-0.3612, 0.0073
Week 96						
n	1	1	3	3	4	4
Mean	0.14740	-0.03720	0.10397	-0.15907	0.11483	-0.12860
(SD)	(NA)	(NA)	(0.032162)	(0.147447)	(0.034077)	(0.134932)
Median	0.14740	-0.03720	0.10870	-0.11120	0.12110	-0.07635
Min, Max	0.1474, 0.1474	-0.0372, -0.0372	0.0697, 0.1335	-0.3245, -0.0415	0.0697, 0.1474	-0.3245, -0.0372
Week 104						
n	0	0	1	1	1	1
Mean			0.18960	-0.05510	0.18960	-0.05510
(SD)			(NA)	(NA)	(NA)	(NA)
Median			0.18960	-0.05510	0.18960	-0.05510
Min, Max			0.1896, 0.1896	-0.0551, -0.0551	0.1896, 0.1896	-0.0551, -0.0551

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.9

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		4		11	
Mean	0.31571		0.22235		0.28176	
(SD)	(0.149896)		(0.085949)		(0.133852)	
Median	0.28500		0.21570		0.25830	
Min, Max	0.1357, 0.5295		0.1332, 0.3248		0.1332, 0.5295	
Week 1						
n	5	5	4	3		
Mean	0.17566	-0.12492	0.26868	0.03797		
(SD)	(0.101274)	(0.054329)	(0.101856)	(0.012001)		
Median	0.15450	-0.13000	0.25970	0.03810		
Min, Max	0.0639, 0.3200	-0.2095, -0.0718	0.1591, 0.3962	0.0259, 0.0499		
Week 2						
n	7	7	5	4		
Mean	0.12430	-0.19141	0.26674	0.01613		
(SD)	(0.057999)	(0.137973)	(0.085046)	(0.038092)		
Median	0.11130	-0.12870	0.27750	0.00620		
Min, Max	0.0608, 0.2100	-0.4371, -0.0700	0.1663, 0.3798	-0.0165, 0.0686		

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Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 3						
n	7	7	4	4		
Mean	0.13123	-0.18449	0.21585	-0.00650		
(SD)	(0.079229)	(0.148080)	(0.109441)	(0.025172)		
Median	0.10000	-0.14000	0.20450	-0.01120		
Min, Max	0.0500, 0.2479	-0.4571, -0.0600	0.1013, 0.3531	-0.0319, 0.0283		
Week 4						
n	6	6	3	3		
Mean	0.14222	-0.14160	0.16963	-0.01857		
(SD)	(0.063174)	(0.091560)	(0.056643)	(0.026806)		
Median	0.12500	-0.11210	0.18430	-0.02610		
Min, Max	0.0771, 0.2300	-0.2995, -0.0586	0.1071, 0.2175	-0.0408, 0.0112		
Week 6						
n	6	6	4	4		
Mean	0.15465	-0.12917	0.23258	0.01023		
(SD)	(0.083927)	(0.068215)	(0.110885)	(0.034384)		
Median	0.13100	-0.13160	0.22315	0.00745		
Min, Max	0.0804, 0.3100	-0.2195, -0.0500	0.1089, 0.3751	-0.0243, 0.0503		

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Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	4	4	4	4		
Mean	0.12635	-0.17120	0.19815	-0.02420		
(SD)	(0.071177)	(0.100516)	(0.066011)	(0.025537)		
Median	0.10210	-0.16040	0.20515	-0.01785		
Min, Max	0.0712, 0.2300	-0.2995, -0.0645	0.1179, 0.2644	-0.0604, -0.0007		
Week 10						
n	5	5	4	4		
Mean	0.12650	-0.16960	0.21198	-0.01038		
(SD)	(0.059335)	(0.080052)	(0.082873)	(0.019469)		
Median	0.10270	-0.17400	0.21375	-0.01190		
Min, Max	0.0768, 0.2243	-0.2828, -0.0589	0.1274, 0.2930	-0.0318, 0.0141		
Week 12						
n	6	6	3	3		
Mean	0.14987	-0.18513	0.19397	-0.01640		
(SD)	(0.075942)	(0.095156)	(0.085096)	(0.016204)		
Median	0.11575	-0.18775	0.16000	-0.01310		
Min, Max	0.0871, 0.2900	-0.3238, -0.0486	0.1311, 0.2908	-0.0340, -0.0021		

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Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 13						
n	5	5	5	4		
Mean	0.17674	-0.10130	0.17704	-0.07920		
(SD)	(0.081900)	(0.090993)	(0.083531)	(0.080711)		
Median	0.13650	-0.10350	0.15850	-0.08230		
Min, Max	0.1099, 0.3100	-0.2195, 0.0000	0.0911, 0.3126	-0.1663, 0.0141		
Week 14						
n	5	5	4	4		
Mean	0.18352	-0.09452	0.11260	-0.10975		
(SD)	(0.063684)	(0.106226)	(0.034018)	(0.079076)		
Median	0.19720	-0.04280	0.11905	-0.10370		
Min, Max	0.1057, 0.2720	-0.2575, 0.0018	0.0655, 0.1468	-0.2053, -0.0263		
Week 15						
n	3	3	5	4		
Mean	0.15040	-0.05650	0.12152	-0.11015		
(SD)	(0.083861)	(0.101266)	(0.028845)	(0.083053)		
Median	0.12620	-0.05440	0.12880	-0.09000		
Min, Max	0.0813, 0.2437	-0.1588, 0.0437	0.0827, 0.1588	-0.2196, -0.0410		

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Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	1	1	4	3		
Mean	0.14910	-0.13590	0.10273	-0.14457		
(SD)	(NA)	(NA)	(0.027807)	(0.077293)		
Median	0.14910	-0.13590	0.10910	-0.14160		
Min, Max	0.1491, 0.1491	-0.1359, -0.1359	0.0644, 0.1283	-0.2233, -0.0688		
Week 18						
n	4	4	4	3		
Mean	0.16300	-0.05218	0.13085	-0.14353		
(SD)	(0.078190)	(0.105907)	(0.078902)	(0.055796)		
Median	0.12600	-0.06770	0.11740	-0.15680		
Min, Max	0.1200, 0.2800	-0.1533, 0.0800	0.0509, 0.2377	-0.1915, -0.0823		
Week 20						
n	4	4	4	3		
Mean	0.15758	-0.13998	0.14550	-0.08663		
(SD)	(0.091430)	(0.076838)	(0.093910)	(0.042587)		
Median	0.12950	-0.13300	0.12500	-0.07850		
Min, Max	0.0813, 0.2900	-0.2395, -0.0544	0.0547, 0.2773	-0.1327, -0.0487		

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Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 22						
n	5	5	4	3		
Mean	0.15832	-0.07636	0.13765	-0.07493		
(SD)	(0.023867)	(0.061548)	(0.052990)	(0.039923)		
Median	0.16000	-0.06000	0.12635	-0.05820		
Min, Max	0.1328, 0.1834	-0.1496, -0.0029	0.0871, 0.2108	-0.1205, -0.0461		
Week 24						
n	6	6	1	1		
Mean	0.19757	-0.08625	0.16570	-0.09260		
(SD)	(0.100255)	(0.055181)	(NA)	(NA)		
Median	0.17500	-0.05915	0.16570	-0.09260		
Min, Max	0.0893, 0.3700	-0.1595, -0.0400	0.1657, 0.1657	-0.0926, -0.0926		
Week 28						
n	4	4	4	3	8	7
Mean	0.22135	-0.09228	0.13005	-0.07727	0.17570	-0.08584
(SD)	(0.168967)	(0.062880)	(0.039534)	(0.061156)	(0.123641)	(0.057341)
Median	0.15770	-0.09975	0.11800	-0.07630	0.12740	-0.07630
Min, Max	0.1000, 0.4700	-0.1496, -0.0200	0.0968, 0.1874	-0.1389, -0.0166	0.0968, 0.4700	-0.1496, -0.0166

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.9

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 32						
n	5	5	3	2	8	7
Mean	0.16270	-0.11534	0.13240	-0.10195	0.15134	-0.11151
(SD)	(0.077250)	(0.097613)	(0.032327)	(0.062013)	(0.062885)	(0.083880)
Median	0.16460	-0.12040	0.11500	-0.10195	0.13980	-0.12040
Min, Max	0.0787, 0.2700	-0.2595, 0.0000	0.1125, 0.1697	-0.1458, -0.0581	0.0787, 0.2700	-0.2595, 0.0000
Week 36						
n	4	4	3	3	7	7
Mean	0.17778	-0.15403	0.10013	-0.08807	0.14450	-0.12576
(SD)	(0.063052)	(0.108940)	(0.006358)	(0.059105)	(0.061021)	(0.091331)
Median	0.18245	-0.15830	0.10350	-0.06960	0.10410	-0.12780
Min, Max	0.0962, 0.2500	-0.2795, -0.0200	0.0928, 0.1041	-0.1542, -0.0404	0.0928, 0.2500	-0.2795, -0.0200
Week 40						
n	4	4	1	1	5	5
Mean	0.20275	-0.08480	0.11090	-0.14740	0.18438	-0.09732
(SD)	(0.068842)	(0.117734)	(NA)	(NA)	(0.072400)	(0.105734)
Median	0.20535	-0.05640	0.11090	-0.14740	0.19070	-0.09430
Min, Max	0.1172, 0.2831	-0.2464, 0.0200	0.1109, 0.1109	-0.1474, -0.1474	0.1109, 0.2831	-0.2464, 0.0200

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.9

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 44						
n	5	5	2	2	7	7
Mean	0.18596	-0.10662	0.10455	-0.09120	0.16270	-0.10221
(SD)	(0.081490)	(0.079066)	(0.003889)	(0.084570)	(0.077509)	(0.073595)
Median	0.16540	-0.12400	0.10455	-0.09120	0.16100	-0.12400
Min, Max	0.1018, 0.3216	-0.2079, -0.0200	0.1018, 0.1073	-0.1510, -0.0314	0.1018, 0.3216	-0.2079, -0.0200
Week 48						
n	5	5	3	2	8	7
Mean	0.15000	-0.08468	0.16807	-0.09965	0.15678	-0.08896
(SD)	(0.045204)	(0.059429)	(0.097492)	(0.007566)	(0.063014)	(0.049167)
Median	0.12930	-0.09910	0.15330	-0.09965	0.14130	-0.09910
Min, Max	0.1051, 0.2136	-0.1630, -0.0200	0.0788, 0.2721	-0.1050, -0.0943	0.0788, 0.2721	-0.1630, -0.0200
Week 52						
n	3	3	3	2	6	5
Mean	0.14913	-0.09253	0.11333	-0.11105	0.13123	-0.09994
(SD)	(0.026910)	(0.065743)	(0.018545)	(0.044901)	(0.028491)	(0.052612)
Median	0.13680	-0.10940	0.11550	-0.11105	0.13065	-0.10940
Min, Max	0.1306, 0.1800	-0.1482, -0.0200	0.0938, 0.1307	-0.1428, -0.0793	0.0938, 0.1800	-0.1482, -0.0200

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.9

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 56						
n	5	5	2	1	7	6
Mean	0.16796	-0.11008	0.14445	-0.13430	0.16124	-0.11412
(SD)	(0.083433)	(0.101991)	(0.028921)	(NA)	(0.070083)	(0.091758)
Median	0.19000	-0.09180	0.14445	-0.13430	0.16490	-0.10195
Min, Max	0.0439, 0.2506	-0.2789, -0.0100	0.1240, 0.1649	-0.1343, -0.1343	0.0439, 0.2506	-0.2789, -0.0100
Week 60						
n	3	3	3	3	6	6
Mean	0.21197	-0.12620	0.12173	-0.06647	0.16685	-0.09633
(SD)	(0.055250)	(0.117085)	(0.014268)	(0.077787)	(0.061197)	(0.094733)
Median	0.20590	-0.07910	0.12090	-0.05220	0.14820	-0.06565
Min, Max	0.1600, 0.2700	-0.2595, -0.0400	0.1079, 0.1364	-0.1504, 0.0032	0.1079, 0.2700	-0.2595, 0.0032
Week 64						
n	3	3	1	1	4	4
Mean	0.17523	-0.16293	0.11080	-0.06230	0.15913	-0.13778
(SD)	(0.156013)	(0.015082)	(NA)	(NA)	(0.131395)	(0.051802)
Median	0.12570	-0.15930	0.11080	-0.06230	0.11825	-0.15465
Min, Max	0.0500, 0.3500	-0.1795, -0.1500	0.1108, 0.1108	-0.0623, -0.0623	0.0500, 0.3500	-0.1795, -0.0623

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADLB

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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.9

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 68						
n	2	2	2	2	4	4
Mean	0.10985	-0.13265	0.08925	-0.06390	0.09955	-0.09828
(SD)	(0.040093)	(0.020011)	(0.004455)	(0.023759)	(0.026151)	(0.043556)
Median	0.10985	-0.13265	0.08925	-0.06390	0.08925	-0.09960
Min, Max	0.0815, 0.1382	-0.1468, -0.1185	0.0861, 0.0924	-0.0807, -0.0471	0.0815, 0.1382	-0.1468, -0.0471
Week 72						
n	3	3	1	1	4	4
Mean	0.23827	-0.09990	0.10320	-0.06990	0.20450	-0.09240
(SD)	(0.094069)	(0.117538)	(NA)	(NA)	(0.102275)	(0.097134)
Median	0.28480	-0.07000	0.10320	-0.06990	0.20740	-0.06995
Min, Max	0.1300, 0.3000	-0.2295, -0.0002	0.1032, 0.1032	-0.0699, -0.0699	0.1032, 0.3000	-0.2295, -0.0002
Week 80						
n	2	2	0	0	2	2
Mean	0.28500	-0.07975			0.28500	-0.07975
(SD)	(0.091924)	(0.141068)			(0.091924)	(0.141068)
Median	0.28500	-0.07975			0.28500	-0.07975
Min, Max	0.2200, 0.3500	-0.1795, 0.0200			0.2200, 0.3500	-0.1795, 0.0200

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Source: ADaM.ADSL, ADaM.ADLB
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Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
ALXN2040-PNH-301

Table GERMANY.2.6.4.9

Summary of Actual Values and Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	1	1	0	0	1	1
Mean	0.21550	-0.06950			0.21550	-0.06950
(SD)	(NA)	(NA)			(NA)	(NA)
Median	0.21550	-0.06950			0.21550	-0.06950
Min, Max	0.2155, 0.2155	-0.0695, -0.0695			0.2155, 0.2155	-0.0695, -0.0695
Week 96						
n	1	1	0	0	1	1
Mean	0.18940	-0.09560			0.18940	-0.09560
(SD)	(NA)	(NA)			(NA)	(NA)
Median	0.18940	-0.09560			0.18940	-0.09560
Min, Max	0.1894, 0.1894	-0.0956, -0.0956			0.1894, 0.1894	-0.0956, -0.0956
Week 104						
n	1	1	1	1	2	2
Mean	0.17020	-0.11480	0.07600	-0.05720	0.12310	-0.08600
(SD)	(NA)	(NA)	(NA)	(NA)	(0.066609)	(0.040729)
Median	0.17020	-0.11480	0.07600	-0.05720	0.12310	-0.08600
Min, Max	0.1702, 0.1702	-0.1148, -0.1148	0.0760, 0.0760	-0.0572, -0.0572	0.0760, 0.1702	-0.1148, -0.0572

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Source: ADaM.ADSL, ADaM.ADLB

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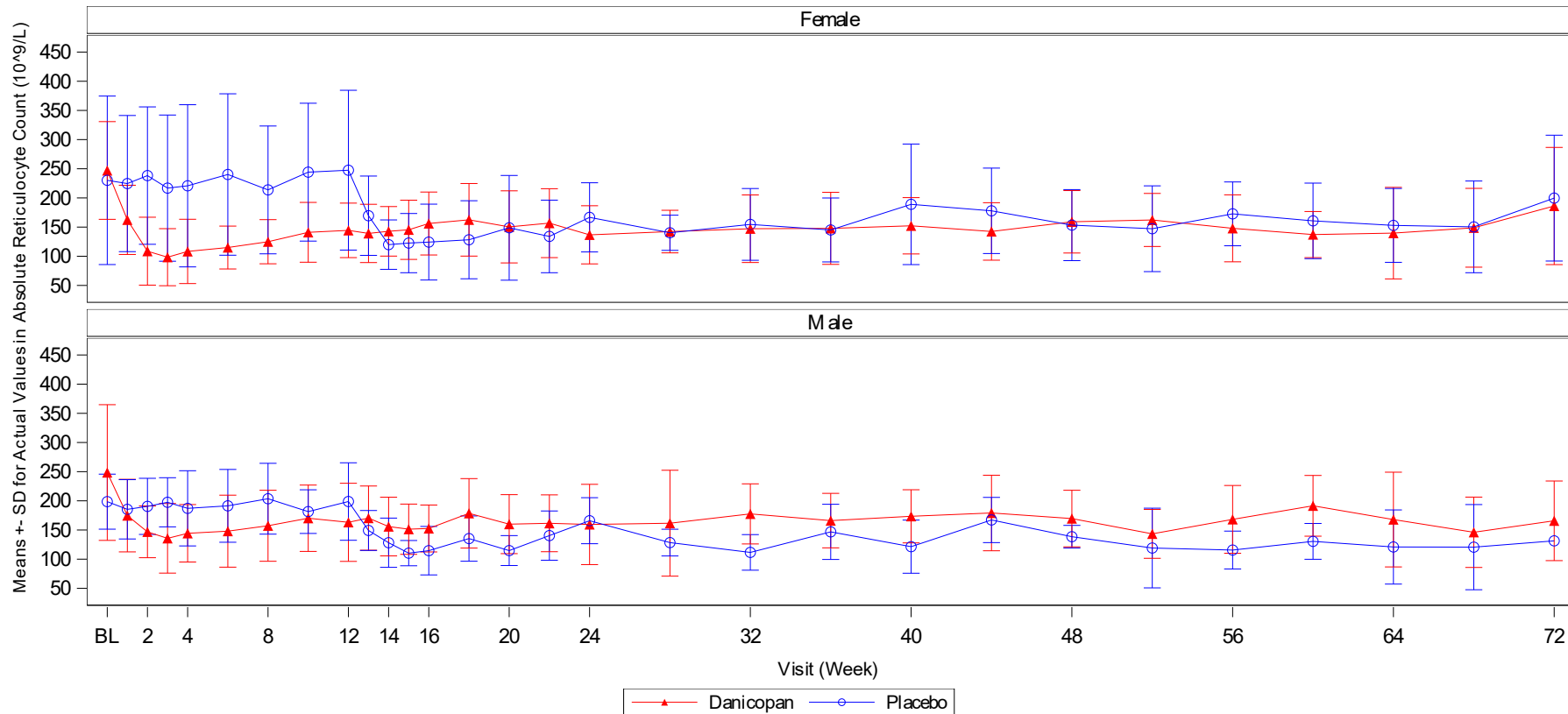
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Alexion Pharmaceuticals, Inc.
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Figure GERMANY.2.2.2.3.2
Plots of Mean \pm SD for Actual Values in Absolute Reticulocyte Count ($10^9/L$) During 72 Weeks by Sex
Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADLB

Run Date: 2024-04-26T15:31:05

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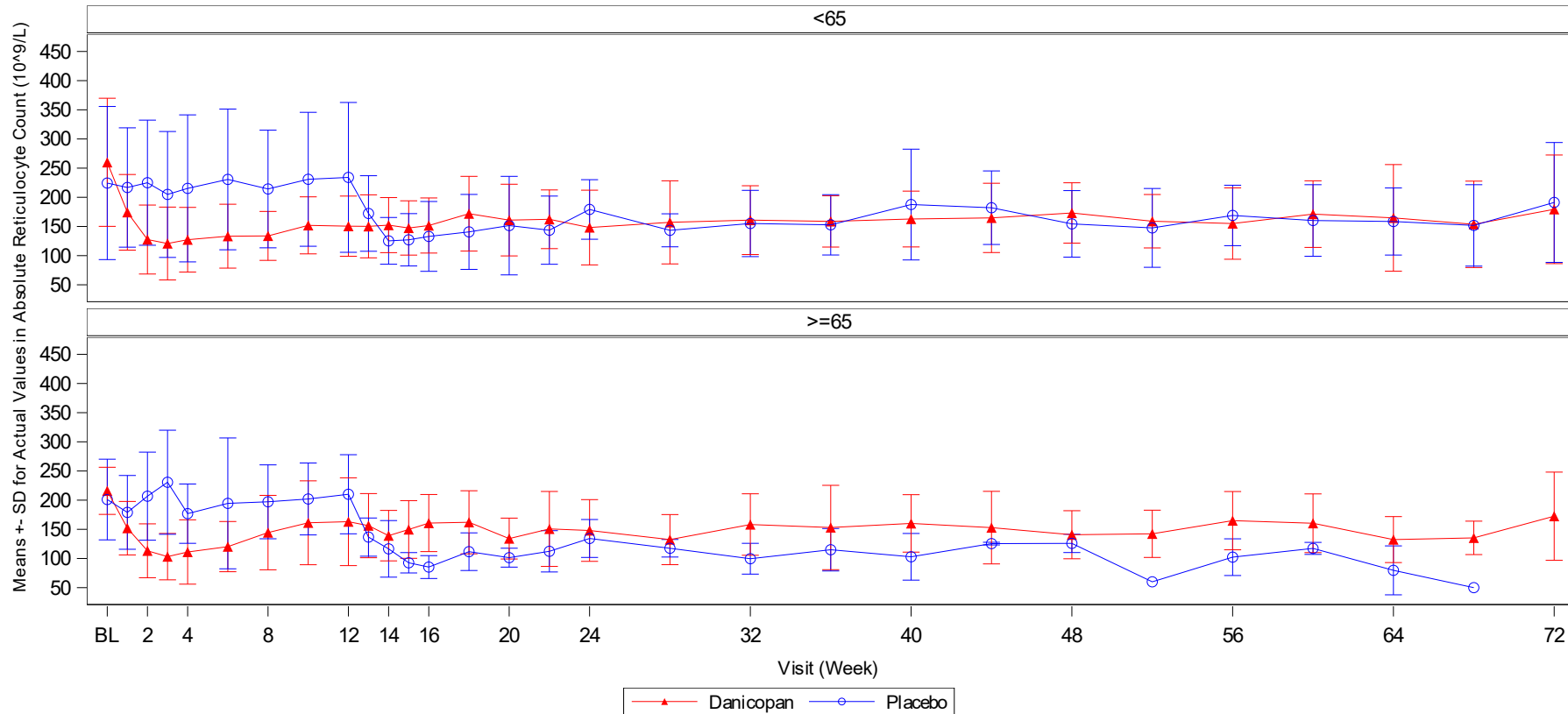
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Figure GERMANY.2.2.2.3.3
Plots of Mean \pm SD for Actual Values in Absolute Reticulocyte Count ($10^9/L$) During 72 Weeks by Age at Informed Consent (< 65 and \geq 65 Years)
Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADLB

Run Date: 2024-04-26T15:31:05

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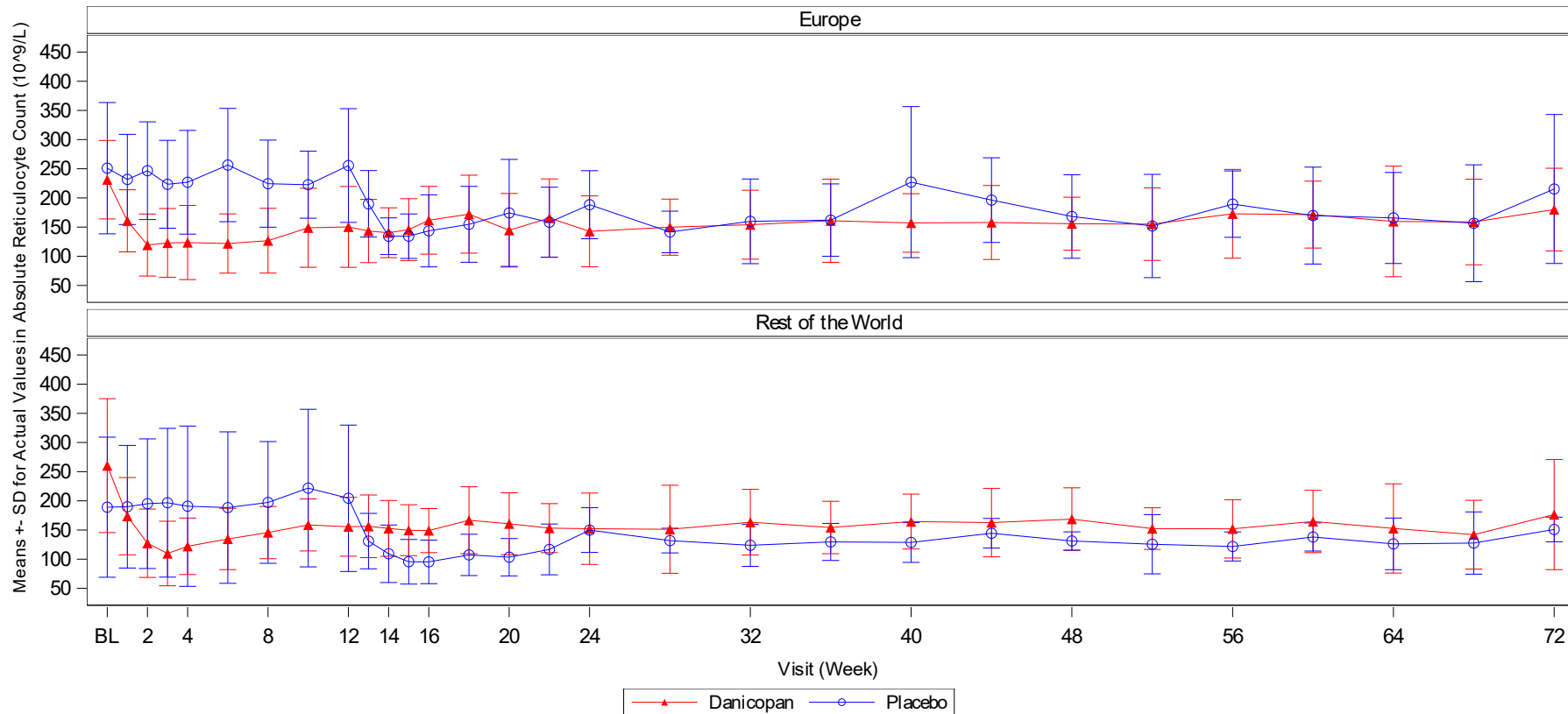
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Figure GERMANY.2.2.2.3.4
Plots of Mean \pm SD for Actual Values in Absolute Reticulocyte Count ($10^9/L$) During 72 Weeks by Region
Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADLB

Run Date: 2024-04-26T15:31:06

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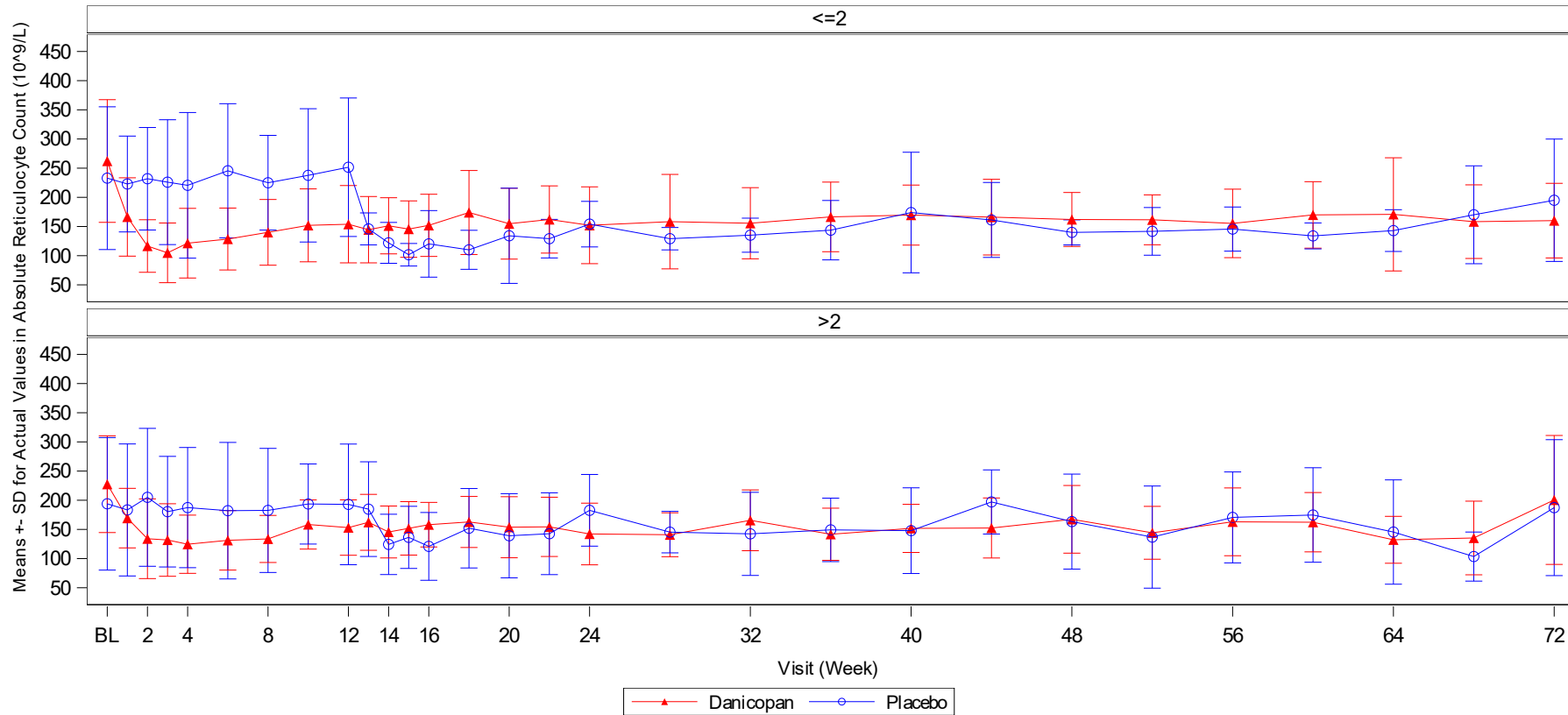
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Figure GERMANY.2.2.2.3.5

Plots of Mean \pm SD for Actual Values in Absolute Reticulocyte Count ($10^9/L$) During 72 Weeks by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADLB

Run Date: 2024-04-26T15:31:06

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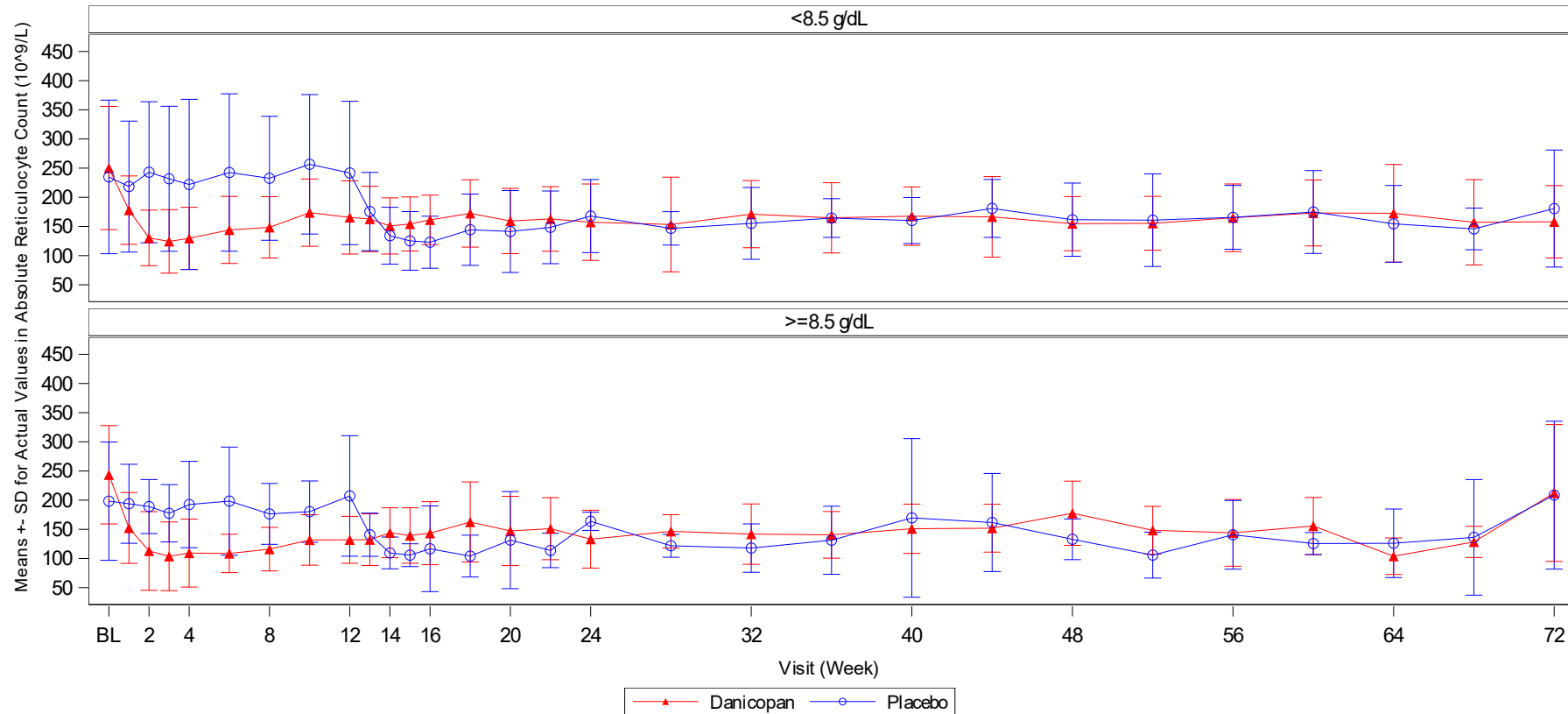
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Figure GERMANY.2.2.2.3.6

Plots of Mean \pm SD for Actual Values in Absolute Reticulocyte Count ($10^9/L$) During 72 Weeks by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADLB

Run Date: 2024-04-26T15:31:07

Data cutoff date of 31Mar2023

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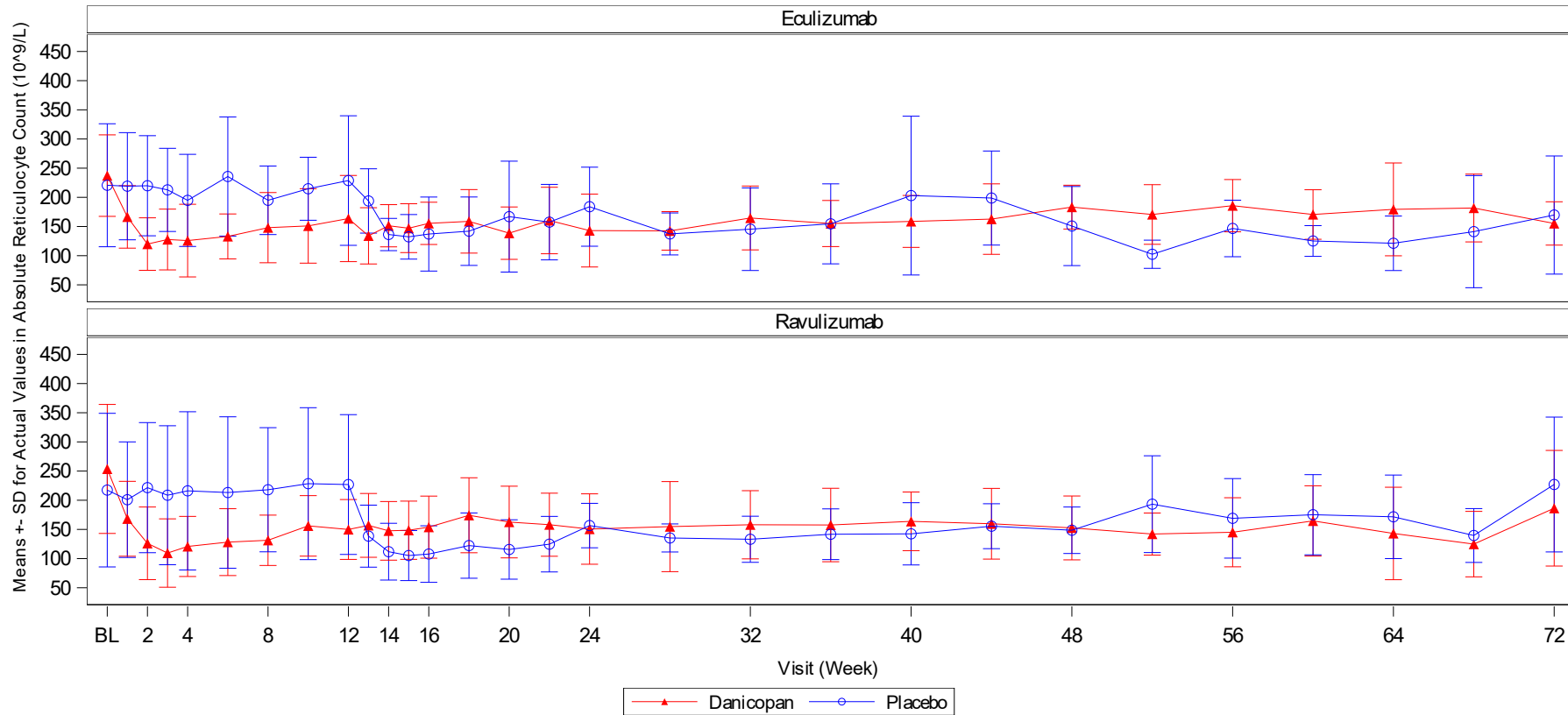
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Figure GERMANY.2.2.2.3.7
Plots of Mean +/- SD for Actual Values in Absolute Reticulocyte Count ($10^9/L$) During 72 Weeks
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADLB

Run Date: 2024-04-26T15:31:07

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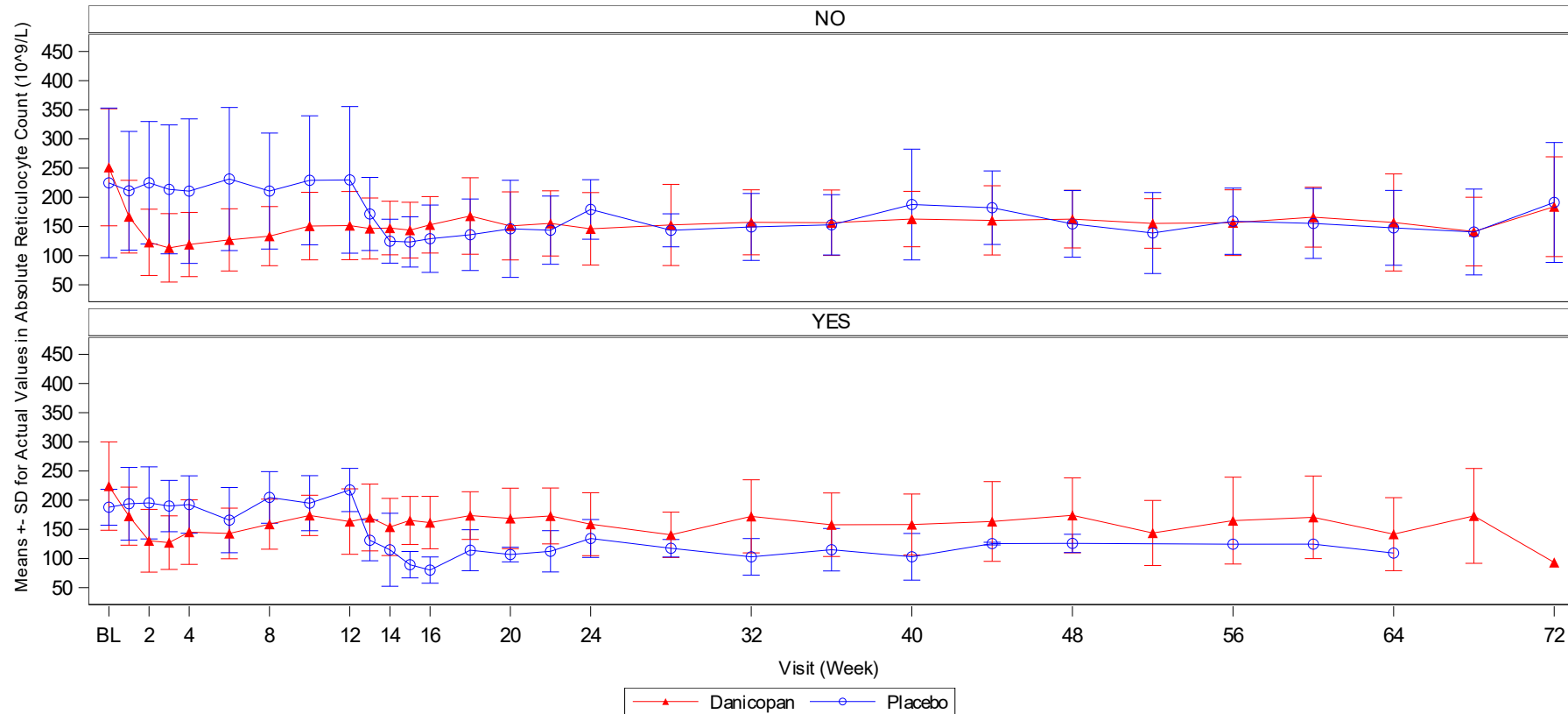
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Figure GERMANY.2.2.2.3.8

Plots of Mean \pm SD for Actual Values in Absolute Reticulocyte Count ($10^9/L$) During 72 Weeks by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADLB

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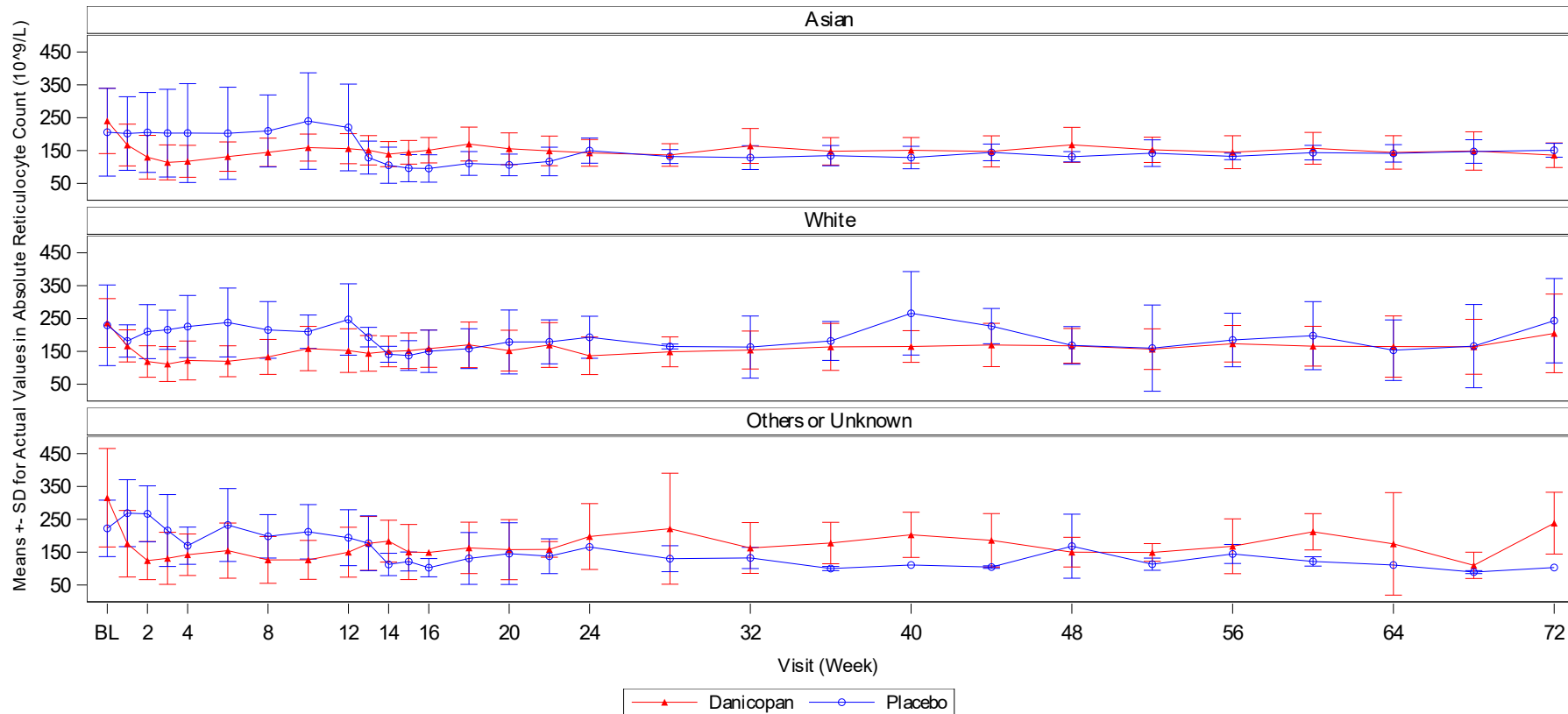
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Post-hoc Analysen Studie ALPHA

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ALXN2040-PNH-301

Figure GERMANY.2.2.2.3.9
Plots of Mean \pm SD for Actual Values in Absolute Reticulocyte Count ($10^9/L$) During 72 Weeks by Race
Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADLB

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Table GERMANY.2.2.4.1.1
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-0.0762 (0.01104)	-0.0456 (0.01719)	-0.0306 (0.02051)	0.1494
	95% CI for LS MEAN	(-0.0991, -0.0534)	(-0.0813, -0.0099)	(-0.0732, 0.0119)	
Week 2	LS Mean (SEM)	-0.1120 (0.01201)	-0.0350 (0.01963)	-0.0770 (0.02316)	0.0024
	95% CI for LS MEAN	(-0.1366, -0.0874)	(-0.0752, 0.0052)	(-0.1244, -0.0296)	
Week 3	LS Mean (SEM)	-0.1152 (0.01372)	-0.0274 (0.02242)	-0.0878 (0.02648)	0.0025
	95% CI for LS MEAN	(-0.1433, -0.0871)	(-0.0733, 0.0185)	(-0.1420, -0.0336)	
Week 4	LS Mean (SEM)	-0.1102 (0.01248)	-0.0391 (0.02050)	-0.0711 (0.02411)	0.0064
	95% CI for LS MEAN	(-0.1359, -0.0846)	(-0.0811, 0.0029)	(-0.1206, -0.0217)	
Week 6	LS Mean (SEM)	-0.1026 (0.01049)	-0.0330 (0.01694)	-0.0695 (0.02005)	0.0019
	95% CI for LS MEAN	(-0.1242, -0.0810)	(-0.0679, 0.0018)	(-0.1108, -0.0283)	
Week 8	LS Mean (SEM)	-0.0891 (0.01117)	-0.0275 (0.01663)	-0.0616 (0.01996)	0.0048
	95% CI for LS MEAN	(-0.1120, -0.0662)	(-0.0617, 0.0068)	(-0.1027, -0.0206)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

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Data cutoff date of 31Mar2023

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Table GERMANY.2.2.4.1.1
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-0.0880 (0.01165)	-0.0361 (0.01897)	-0.0518 (0.02259)	0.0295
	95% CI for LS MEAN	(-0.1118, -0.0641)	(-0.0750, 0.0028)	(-0.0981, -0.0056)	
Week 12	LS Mean (SEM)	-0.0870 (0.01266)	-0.0322 (0.02027)	-0.0548 (0.02399)	0.0298
	95% CI for LS MEAN	(-0.1129, -0.0611)	(-0.0737, 0.0092)	(-0.1038, -0.0058)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:53:29

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.4.1.1
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-0.0779 (0.01022)	-0.0001 (0.01560)	-0.0778 (0.01844)	<0.0001
	95% CI for LS MEAN	(-0.0982, -0.0576)	(-0.0311, 0.0308)	(-0.1144, -0.0412)	
Week 2	LS Mean (SEM)	-0.1337 (0.00982)	0.0040 (0.01501)	-0.1377 (0.01772)	<0.0001
	95% CI for LS MEAN	(-0.1533, -0.1142)	(-0.0259, 0.0338)	(-0.1729, -0.1025)	
Week 3	LS Mean (SEM)	-0.1443 (0.01031)	-0.0083 (0.01504)	-0.1361 (0.01802)	<0.0001
	95% CI for LS MEAN	(-0.1648, -0.1239)	(-0.0382, 0.0216)	(-0.1718, -0.1003)	
Week 4	LS Mean (SEM)	-0.1331 (0.00997)	-0.0086 (0.01502)	-0.1245 (0.01779)	<0.0001
	95% CI for LS MEAN	(-0.1530, -0.1133)	(-0.0385, 0.0212)	(-0.1598, -0.0891)	
Week 6	LS Mean (SEM)	-0.1286 (0.01027)	0.0032 (0.01486)	-0.1318 (0.01784)	<0.0001
	95% CI for LS MEAN	(-0.1490, -0.1082)	(-0.0263, 0.0327)	(-0.1672, -0.0964)	
Week 8	LS Mean (SEM)	-0.1148 (0.01020)	-0.0121 (0.01513)	-0.1027 (0.01801)	<0.0001
	95% CI for LS MEAN	(-0.1351, -0.0946)	(-0.0422, 0.0179)	(-0.1384, -0.0669)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.2.4.1.1
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-0.1015 (0.01100)	0.0042 (0.01501)	-0.1057 (0.01839)	<0.0001
	95% CI for LS MEAN	(-0.1233, -0.0797)	(-0.0256, 0.0340)	(-0.1422, -0.0692)	
Week 12	LS Mean (SEM)	-0.0999 (0.01055)	0.0050 (0.01493)	-0.1049 (0.01808)	<0.0001
	95% CI for LS MEAN	(-0.1208, -0.0790)	(-0.0247, 0.0346)	(-0.1408, -0.0690)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

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Data cutoff date of 31Mar2023

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Table GERMANY.2.2.4.1.1
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value 0.0378

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.4.1.2
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-0.0776 (0.00725)	-0.0075 (0.01103)	-0.0701 (0.01293)	<0.0001
	95% CI for LS MEAN	(-0.0921, -0.0630)	(-0.0296, 0.0147)	(-0.0961, -0.0442)	
Week 2	LS Mean (SEM)	-0.1268 (0.01059)	-0.0062 (0.01637)	-0.1205 (0.01932)	<0.0001
	95% CI for LS MEAN	(-0.1480, -0.1055)	(-0.0390, 0.0266)	(-0.1593, -0.0818)	
Week 3	LS Mean (SEM)	-0.1374 (0.01148)	-0.0168 (0.01730)	-0.1206 (0.02060)	<0.0001
	95% CI for LS MEAN	(-0.1604, -0.1143)	(-0.0515, 0.0179)	(-0.1619, -0.0792)	
Week 4	LS Mean (SEM)	-0.1249 (0.01225)	-0.0157 (0.01824)	-0.1092 (0.02180)	<0.0001
	95% CI for LS MEAN	(-0.1495, -0.1003)	(-0.0524, 0.0210)	(-0.1531, -0.0654)	
Week 6	LS Mean (SEM)	-0.1183 (0.00943)	-0.0017 (0.01387)	-0.1165 (0.01656)	<0.0001
	95% CI for LS MEAN	(-0.1373, -0.0993)	(-0.0297, 0.0262)	(-0.1500, -0.0831)	
Week 8	LS Mean (SEM)	-0.1115 (0.00811)	-0.0084 (0.01158)	-0.1031 (0.01382)	<0.0001
	95% CI for LS MEAN	(-0.1279, -0.0950)	(-0.0319, 0.0151)	(-0.1312, -0.0750)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:53:30

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Table GERMANY.2.2.4.1.2
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-0.1029 (0.00902)	0.0030 (0.01286)	-0.1060 (0.01553)	<0.0001
	95% CI for LS MEAN	(-0.1210, -0.0848)	(-0.0228, 0.0289)	(-0.1372, -0.0748)	
Week 12	LS Mean (SEM)	-0.1030 (0.00976)	-0.0042 (0.01438)	-0.0988 (0.01718)	<0.0001
	95% CI for LS MEAN	(-0.1226, -0.0835)	(-0.0331, 0.0247)	(-0.1333, -0.0643)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.2.4.1.2
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-0.0634 (0.01191)	-0.0031 (0.01959)	-0.0604 (0.02307)	0.0129
	95% CI for LS MEAN	(-0.0876, -0.0392)	(-0.0428, 0.0366)	(-0.1071, -0.0136)	
Week 2	LS Mean (SEM)	-0.1083 (0.01193)	0.0049 (0.01958)	-0.1132 (0.02309)	<0.0001
	95% CI for LS MEAN	(-0.1325, -0.0841)	(-0.0347, 0.0445)	(-0.1600, -0.0664)	
Week 3	LS Mean (SEM)	-0.1185 (0.01197)	0.0132 (0.02092)	-0.1317 (0.02424)	<0.0001
	95% CI for LS MEAN	(-0.1428, -0.0942)	(-0.0289, 0.0554)	(-0.1807, -0.0828)	
Week 4	LS Mean (SEM)	-0.1115 (0.01192)	0.0158 (0.02075)	-0.1273 (0.02414)	<0.0001
	95% CI for LS MEAN	(-0.1357, -0.0873)	(-0.0260, 0.0577)	(-0.1761, -0.0786)	
Week 6	LS Mean (SEM)	-0.0995 (0.01211)	-0.0036 (0.01937)	-0.0960 (0.02301)	0.0002
	95% CI for LS MEAN	(-0.1241, -0.0750)	(-0.0428, 0.0357)	(-0.1426, -0.0493)	
Week 8	LS Mean (SEM)	-0.0804 (0.01184)	-0.0100 (0.01872)	-0.0705 (0.02230)	0.0033
	95% CI for LS MEAN	(-0.1045, -0.0564)	(-0.0480, 0.0281)	(-0.1158, -0.0251)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.
The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.
Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).
P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
Source: ADaM.ADSL, ADaM.ADLB
Run Date: 2024-04-26T15:53:30
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Table GERMANY.2.2.4.1.2
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and \geq 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

\geq 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-0.0651 (0.01282)	-0.0051 (0.01872)	-0.0600 (0.02282)	0.0125
	95% CI for LS MEAN	(-0.0910, -0.0393)	(-0.0432, 0.0330)	(-0.1063, -0.0137)	
Week 12	LS Mean (SEM)	-0.0658 (0.01271)	0.0030 (0.01872)	-0.0687 (0.02279)	0.0047
	95% CI for LS MEAN	(-0.0914, -0.0401)	(-0.0351, 0.0410)	(-0.1150, -0.0225)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.2.4.1.2
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and \geq 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.1217
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.4.1.3
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-0.0697 (0.00793)	0.0062 (0.01307)	-0.0759 (0.01522)	<0.0001
	95% CI for LS MEAN	(-0.0859, -0.0535)	(-0.0203, 0.0327)	(-0.1068, -0.0450)	
Week 2	LS Mean (SEM)	-0.1165 (0.01089)	0.0043 (0.01666)	-0.1207 (0.01980)	<0.0001
	95% CI for LS MEAN	(-0.1387, -0.0943)	(-0.0296, 0.0382)	(-0.1611, -0.0804)	
Week 3	LS Mean (SEM)	-0.1136 (0.01134)	-0.0068 (0.01686)	-0.1068 (0.02020)	<0.0001
	95% CI for LS MEAN	(-0.1370, -0.0903)	(-0.0415, 0.0279)	(-0.1484, -0.0652)	
Week 4	LS Mean (SEM)	-0.1094 (0.01380)	-0.0046 (0.02103)	-0.1048 (0.02505)	0.0002
	95% CI for LS MEAN	(-0.1375, -0.0812)	(-0.0473, 0.0382)	(-0.1558, -0.0538)	
Week 6	LS Mean (SEM)	-0.1100 (0.01162)	0.0137 (0.01680)	-0.1238 (0.02026)	<0.0001
	95% CI for LS MEAN	(-0.1341, -0.0860)	(-0.0211, 0.0486)	(-0.1658, -0.0817)	
Week 8	LS Mean (SEM)	-0.1081 (0.01142)	-0.0004 (0.01557)	-0.1077 (0.01928)	<0.0001
	95% CI for LS MEAN	(-0.1318, -0.0844)	(-0.0328, 0.0320)	(-0.1477, -0.0677)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:53:32

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.4.1.3
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-0.0847 (0.01208)	0.0027 (0.01542)	-0.0874 (0.01957)	0.0002
	95% CI for LS MEAN	(-0.1096, -0.0598)	(-0.0293, 0.0346)	(-0.1278, -0.0469)	
Week 12	LS Mean (SEM)	-0.0859 (0.01320)	0.0028 (0.01739)	-0.0887 (0.02177)	0.0004
	95% CI for LS MEAN	(-0.1129, -0.0588)	(-0.0330, 0.0387)	(-0.1335, -0.0439)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:53:32

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Table GERMANY.2.2.4.1.3
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-0.0786 (0.00926)	-0.0276 (0.01383)	-0.0510 (0.01635)	0.0036
	95% CI for LS MEAN	(-0.0973, -0.0599)	(-0.0557, 0.0005)	(-0.0842, -0.0179)	
Week 2	LS Mean (SEM)	-0.1266 (0.01233)	-0.0175 (0.01999)	-0.1090 (0.02330)	<0.0001
	95% CI for LS MEAN	(-0.1515, -0.1016)	(-0.0580, 0.0229)	(-0.1562, -0.0619)	
Week 3	LS Mean (SEM)	-0.1434 (0.01242)	-0.0237 (0.02007)	-0.1196 (0.02345)	<0.0001
	95% CI for LS MEAN	(-0.1685, -0.1182)	(-0.0644, 0.0169)	(-0.1671, -0.0722)	
Week 4	LS Mean (SEM)	-0.1314 (0.01330)	-0.0229 (0.02140)	-0.1084 (0.02504)	0.0001
	95% CI for LS MEAN	(-0.1583, -0.1044)	(-0.0664, 0.0205)	(-0.1593, -0.0576)	
Week 6	LS Mean (SEM)	-0.1187 (0.01018)	-0.0261 (0.01632)	-0.0926 (0.01898)	<0.0001
	95% CI for LS MEAN	(-0.1393, -0.0981)	(-0.0592, 0.0070)	(-0.1310, -0.0541)	
Week 8	LS Mean (SEM)	-0.1012 (0.01024)	-0.0203 (0.01592)	-0.0810 (0.01867)	0.0002
	95% CI for LS MEAN	(-0.1221, -0.0804)	(-0.0528, 0.0123)	(-0.1191, -0.0428)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:53:32

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Table GERMANY.2.2.4.1.3
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-0.0953 (0.01073)	-0.0109 (0.01635)	-0.0845 (0.01937)	<0.0001
	95% CI for LS MEAN	(-0.1170, -0.0736)	(-0.0440, 0.0223)	(-0.1237, -0.0452)	
Week 12	LS Mean (SEM)	-0.0974 (0.01136)	-0.0131 (0.01801)	-0.0843 (0.02109)	0.0003
	95% CI for LS MEAN	(-0.1203, -0.0745)	(-0.0495, 0.0233)	(-0.1269, -0.0416)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:53:32

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.4.1.3
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value 0.2397

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.4.1.4
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions Within 6 Months of Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-0.0578 (0.01090)	-0.0100 (0.01660)	-0.0478 (0.01988)	0.0234
	95% CI for LS MEAN	(-0.0801, -0.0355)	(-0.0442, 0.0242)	(-0.0886, -0.0070)	
Week 2	LS Mean (SEM)	-0.0934 (0.01405)	-0.0114 (0.02246)	-0.0820 (0.02622)	0.0039
	95% CI for LS MEAN	(-0.1221, -0.0647)	(-0.0573, 0.0344)	(-0.1355, -0.0284)	
Week 3	LS Mean (SEM)	-0.1024 (0.01460)	-0.0198 (0.02309)	-0.0826 (0.02697)	0.0052
	95% CI for LS MEAN	(-0.1323, -0.0724)	(-0.0673, 0.0277)	(-0.1381, -0.0271)	
Week 4	LS Mean (SEM)	-0.0990 (0.01381)	-0.0051 (0.02149)	-0.0939 (0.02522)	0.0010
	95% CI for LS MEAN	(-0.1274, -0.0707)	(-0.0495, 0.0392)	(-0.1459, -0.0419)	
Week 6	LS Mean (SEM)	-0.0964 (0.01506)	-0.0110 (0.02371)	-0.0854 (0.02781)	0.0059
	95% CI for LS MEAN	(-0.1276, -0.0651)	(-0.0604, 0.0384)	(-0.1433, -0.0274)	
Week 8	LS Mean (SEM)	-0.0820 (0.01287)	-0.0116 (0.01939)	-0.0704 (0.02294)	0.0061
	95% CI for LS MEAN	(-0.1087, -0.0554)	(-0.0521, 0.0289)	(-0.1184, -0.0225)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factor of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.2.4.1.4
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions Within 6 Months of Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-0.0768 (0.01090)	-0.0131 (0.01487)	-0.0637 (0.01819)	0.0034
	95% CI for LS MEAN	(-0.0998, -0.0538)	(-0.0451, 0.0189)	(-0.1026, -0.0248)	
Week 12	LS Mean (SEM)	-0.0757 (0.01248)	-0.0116 (0.01885)	-0.0641 (0.02224)	0.0081
	95% CI for LS MEAN	(-0.1013, -0.0502)	(-0.0505, 0.0272)	(-0.1099, -0.0183)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factor of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.2.4.1.4
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions Within 6 Months of Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-0.0830 (0.00794)	-0.0053 (0.01164)	-0.0777 (0.01415)	<0.0001
	95% CI for LS MEAN	(-0.0992, -0.0669)	(-0.0291, 0.0184)	(-0.1065, -0.0489)	
Week 2	LS Mean (SEM)	-0.1387 (0.00914)	-0.0003 (0.01396)	-0.1384 (0.01672)	<0.0001
	95% CI for LS MEAN	(-0.1572, -0.1202)	(-0.0286, 0.0279)	(-0.1722, -0.1045)	
Week 3	LS Mean (SEM)	-0.1492 (0.01011)	-0.0060 (0.01523)	-0.1431 (0.01830)	<0.0001
	95% CI for LS MEAN	(-0.1696, -0.1287)	(-0.0368, 0.0247)	(-0.1801, -0.1062)	
Week 4	LS Mean (SEM)	-0.1355 (0.01304)	-0.0148 (0.01967)	-0.1207 (0.02361)	<0.0001
	95% CI for LS MEAN	(-0.1619, -0.1091)	(-0.0547, 0.0250)	(-0.1685, -0.0728)	
Week 6	LS Mean (SEM)	-0.1252 (0.00800)	0.0014 (0.01168)	-0.1266 (0.01418)	<0.0001
	95% CI for LS MEAN	(-0.1414, -0.1090)	(-0.0222, 0.0251)	(-0.1553, -0.0979)	
Week 8	LS Mean (SEM)	-0.1156 (0.00830)	-0.0068 (0.01161)	-0.1088 (0.01431)	<0.0001
	95% CI for LS MEAN	(-0.1324, -0.0989)	(-0.0303, 0.0166)	(-0.1377, -0.0799)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factor of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:53:33

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Table GERMANY.2.2.4.1.4
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions Within 6 Months of Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-0.1030 (0.01024)	0.0061 (0.01427)	-0.1092 (0.01760)	<0.0001
	95% CI for LS MEAN	(-0.1236, -0.0824)	(-0.0227, 0.0349)	(-0.1446, -0.0737)	
Week 12	LS Mean (SEM)	-0.1057 (0.01125)	0.0003 (0.01591)	-0.1060 (0.01951)	<0.0001
	95% CI for LS MEAN	(-0.1283, -0.0831)	(-0.0317, 0.0324)	(-0.1453, -0.0667)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factor of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.4.1.4
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions Within 6 Months of Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.0565
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factor of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.4.1.5
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-0.0671 (0.00810)	-0.0160 (0.01299)	-0.0511 (0.01540)	0.0020
	95% CI for LS MEAN	(-0.0835, -0.0507)	(-0.0423, 0.0103)	(-0.0822, -0.0199)	
Week 2	LS Mean (SEM)	-0.1170 (0.01124)	-0.0010 (0.01830)	-0.1160 (0.02151)	<0.0001
	95% CI for LS MEAN	(-0.1398, -0.0941)	(-0.0381, 0.0361)	(-0.1596, -0.0724)	
Week 3	LS Mean (SEM)	-0.1286 (0.01203)	-0.0013 (0.01938)	-0.1274 (0.02285)	<0.0001
	95% CI for LS MEAN	(-0.1531, -0.1042)	(-0.0406, 0.0380)	(-0.1737, -0.0810)	
Week 4	LS Mean (SEM)	-0.1193 (0.01283)	-0.0009 (0.02108)	-0.1184 (0.02472)	<0.0001
	95% CI for LS MEAN	(-0.1453, -0.0933)	(-0.0435, 0.0416)	(-0.1683, -0.0684)	
Week 6	LS Mean (SEM)	-0.1014 (0.01042)	0.0084 (0.01675)	-0.1098 (0.01976)	<0.0001
	95% CI for LS MEAN	(-0.1226, -0.0803)	(-0.0255, 0.0423)	(-0.1499, -0.0698)	
Week 8	LS Mean (SEM)	-0.0957 (0.00984)	-0.0024 (0.01525)	-0.0933 (0.01819)	<0.0001
	95% CI for LS MEAN	(-0.1158, -0.0756)	(-0.0336, 0.0288)	(-0.1305, -0.0561)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.2.4.1.5
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-0.0793 (0.01105)	0.0064 (0.01613)	-0.0857 (0.01959)	<0.0001
	95% CI for LS MEAN	(-0.1016, -0.0571)	(-0.0262, 0.0390)	(-0.1253, -0.0462)	
Week 12	LS Mean (SEM)	-0.0845 (0.01174)	-0.0012 (0.01818)	-0.0832 (0.02168)	0.0004
	95% CI for LS MEAN	(-0.1082, -0.0608)	(-0.0380, 0.0355)	(-0.1270, -0.0394)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.2.4.1.5
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-0.0840 (0.00899)	-0.0006 (0.01248)	-0.0834 (0.01524)	<0.0001
	95% CI for LS MEAN	(-0.1024, -0.0656)	(-0.0263, 0.0251)	(-0.1147, -0.0521)	
Week 2	LS Mean (SEM)	-0.1277 (0.01188)	-0.0043 (0.01760)	-0.1233 (0.02108)	<0.0001
	95% CI for LS MEAN	(-0.1519, -0.1034)	(-0.0403, 0.0316)	(-0.1664, -0.0803)	
Week 3	LS Mean (SEM)	-0.1369 (0.01205)	-0.0227 (0.01800)	-0.1142 (0.02147)	<0.0001
	95% CI for LS MEAN	(-0.1616, -0.1122)	(-0.0595, 0.0142)	(-0.1582, -0.0703)	
Week 4	LS Mean (SEM)	-0.1340 (0.01480)	-0.0232 (0.02014)	-0.1108 (0.02462)	0.0003
	95% CI for LS MEAN	(-0.1649, -0.1031)	(-0.0655, 0.0191)	(-0.1625, -0.0591)	
Week 6	LS Mean (SEM)	-0.1327 (0.01013)	-0.0174 (0.01379)	-0.1153 (0.01668)	<0.0001
	95% CI for LS MEAN	(-0.1536, -0.1118)	(-0.0460, 0.0113)	(-0.1500, -0.0807)	
Week 8	LS Mean (SEM)	-0.1207 (0.01004)	-0.0170 (0.01291)	-0.1037 (0.01591)	<0.0001
	95% CI for LS MEAN	(-0.1415, -0.0999)	(-0.0440, 0.0100)	(-0.1369, -0.0705)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.2.4.1.5
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-0.1093 (0.01017)	-0.0123 (0.01346)	-0.0970 (0.01653)	<0.0001
	95% CI for LS MEAN	(-0.1303, -0.0883)	(-0.0402, 0.0157)	(-0.1313, -0.0628)	
Week 12	LS Mean (SEM)	-0.1179 (0.01204)	-0.0020 (0.01616)	-0.1159 (0.01993)	<0.0001
	95% CI for LS MEAN	(-0.1432, -0.0926)	(-0.0363, 0.0324)	(-0.1582, -0.0737)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.2.4.1.5
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1 by the Randomization
Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.3768
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.4.1.6
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-0.0591 (0.01351)	0.0081 (0.01978)	-0.0672 (0.02367)	0.0059
	95% CI for LS MEAN	(-0.0861, -0.0322)	(-0.0313, 0.0475)	(-0.1144, -0.0201)	
Week 2	LS Mean (SEM)	-0.1136 (0.01288)	-0.0017 (0.01949)	-0.1119 (0.02305)	<0.0001
	95% CI for LS MEAN	(-0.1394, -0.0878)	(-0.0406, 0.0372)	(-0.1579, -0.0659)	
Week 3	LS Mean (SEM)	-0.1133 (0.01391)	-0.0010 (0.02040)	-0.1123 (0.02434)	<0.0001
	95% CI for LS MEAN	(-0.1410, -0.0856)	(-0.0417, 0.0396)	(-0.1607, -0.0638)	
Week 4	LS Mean (SEM)	-0.1084 (0.01306)	-0.0172 (0.01958)	-0.0912 (0.02323)	0.0002
	95% CI for LS MEAN	(-0.1346, -0.0823)	(-0.0563, 0.0219)	(-0.1376, -0.0449)	
Week 6	LS Mean (SEM)	-0.1055 (0.01358)	0.0094 (0.01896)	-0.1150 (0.02299)	<0.0001
	95% CI for LS MEAN	(-0.1326, -0.0784)	(-0.0285, 0.0473)	(-0.1609, -0.0691)	
Week 8	LS Mean (SEM)	-0.0872 (0.01359)	-0.0115 (0.01923)	-0.0756 (0.02323)	0.0018
	95% CI for LS MEAN	(-0.1143, -0.0600)	(-0.0499, 0.0269)	(-0.1220, -0.0293)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.2.4.1.6
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-0.0948 (0.01438)	0.0065 (0.01878)	-0.1013 (0.02337)	<0.0001
	95% CI for LS MEAN	(-0.1235, -0.0662)	(-0.0311, 0.0440)	(-0.1479, -0.0547)	
Week 12	LS Mean (SEM)	-0.0814 (0.01499)	-0.0023 (0.01935)	-0.0791 (0.02420)	0.0016
	95% CI for LS MEAN	(-0.1112, -0.0516)	(-0.0410, 0.0363)	(-0.1273, -0.0309)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.2.4.1.6
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-0.0808 (0.00763)	-0.0163 (0.01190)	-0.0645 (0.01393)	<0.0001
	95% CI for LS MEAN	(-0.0962, -0.0654)	(-0.0403, 0.0077)	(-0.0926, -0.0364)	
Week 2	LS Mean (SEM)	-0.1251 (0.01137)	-0.0054 (0.01813)	-0.1198 (0.02124)	<0.0001
	95% CI for LS MEAN	(-0.1480, -0.1022)	(-0.0419, 0.0312)	(-0.1626, -0.0769)	
Week 3	LS Mean (SEM)	-0.1386 (0.01130)	-0.0188 (0.01798)	-0.1198 (0.02108)	<0.0001
	95% CI for LS MEAN	(-0.1614, -0.1158)	(-0.0550, 0.0175)	(-0.1624, -0.0773)	
Week 4	LS Mean (SEM)	-0.1270 (0.01127)	-0.0115 (0.01785)	-0.1155 (0.02096)	<0.0001
	95% CI for LS MEAN	(-0.1498, -0.1043)	(-0.0476, 0.0245)	(-0.1579, -0.0732)	
Week 6	LS Mean (SEM)	-0.1171 (0.00961)	-0.0143 (0.01523)	-0.1028 (0.01784)	<0.0001
	95% CI for LS MEAN	(-0.1365, -0.0977)	(-0.0451, 0.0165)	(-0.1389, -0.0668)	
Week 8	LS Mean (SEM)	-0.1104 (0.00847)	-0.0109 (0.01287)	-0.0994 (0.01516)	<0.0001
	95% CI for LS MEAN	(-0.1275, -0.0932)	(-0.0372, 0.0153)	(-0.1303, -0.0685)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.
The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.
Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).
P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.
Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB
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Table GERMANY.2.2.4.1.6
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-0.0897 (0.00986)	-0.0103 (0.01526)	-0.0794 (0.01802)	<0.0001
	95% CI for LS MEAN	(-0.1096, -0.0698)	(-0.0410, 0.0205)	(-0.1157, -0.0431)	
Week 12	LS Mean (SEM)	-0.0982 (0.01003)	-0.0020 (0.01581)	-0.0962 (0.01857)	<0.0001
	95% CI for LS MEAN	(-0.1184, -0.0780)	(-0.0339, 0.0298)	(-0.1336, -0.0587)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.2.4.1.6
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.8813
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.4.1.7
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-0.0820 (0.04322)	-0.0100 (0.03143)	-0.0719 (0.06422)	0.2959
	95% CI for LS MEAN	(-0.1823, 0.0184)	(-0.0807, 0.0606)	(-0.2206, 0.0767)	
Week 2	LS Mean (SEM)	-0.1252 (0.04318)	0.0011 (0.03253)	-0.1263 (0.06498)	0.0872
	95% CI for LS MEAN	(-0.2255, -0.0249)	(-0.0709, 0.0732)	(-0.2758, 0.0231)	
Week 3	LS Mean (SEM)	-0.1274 (0.04324)	0.0018 (0.03256)	-0.1291 (0.06509)	0.0818
	95% CI for LS MEAN	(-0.2277, -0.0270)	(-0.0704, 0.0739)	(-0.2787, 0.0205)	
Week 4	LS Mean (SEM)	-0.1107 (0.04319)	-0.0006 (0.03283)	-0.1100 (0.06527)	0.1294
	95% CI for LS MEAN	(-0.2110, -0.0104)	(-0.0732, 0.0719)	(-0.2598, 0.0398)	
Week 6	LS Mean (SEM)	-0.1113 (0.04297)	-0.0281 (0.03280)	-0.0833 (0.06507)	0.2360
	95% CI for LS MEAN	(-0.2115, -0.0112)	(-0.1006, 0.0444)	(-0.2329, 0.0664)	
Week 8	LS Mean (SEM)	-0.0883 (0.04302)	0.0009 (0.03143)	-0.0891 (0.06397)	0.2023
	95% CI for LS MEAN	(-0.1885, 0.0120)	(-0.0698, 0.0715)	(-0.2376, 0.0593)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

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Table GERMANY.2.2.4.1.7
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-0.0752 (0.04346)	-0.0090 (0.03143)	-0.0662 (0.06440)	0.3346
	95% CI for LS MEAN	(-0.1758, 0.0255)	(-0.0797, 0.0617)	(-0.2150, 0.0827)	
Week 12	LS Mean (SEM)	-0.0909 (0.04297)	0.0136 (0.03143)	-0.1046 (0.06401)	0.1421
	95% CI for LS MEAN	(-0.1911, 0.0092)	(-0.0570, 0.0843)	(-0.2530, 0.0439)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:53:36

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.4.1.7
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-0.0769 (0.00686)	-0.0099 (0.01074)	-0.0670 (0.01255)	<0.0001
	95% CI for LS MEAN	(-0.0906, -0.0631)	(-0.0314, 0.0116)	(-0.0921, -0.0418)	
Week 2	LS Mean (SEM)	-0.1253 (0.00897)	-0.0071 (0.01413)	-0.1183 (0.01657)	<0.0001
	95% CI for LS MEAN	(-0.1432, -0.1074)	(-0.0353, 0.0212)	(-0.1514, -0.0851)	
Week 3	LS Mean (SEM)	-0.1353 (0.00938)	-0.0153 (0.01458)	-0.1200 (0.01718)	<0.0001
	95% CI for LS MEAN	(-0.1541, -0.1165)	(-0.0444, 0.0139)	(-0.1544, -0.0856)	
Week 4	LS Mean (SEM)	-0.1276 (0.01058)	-0.0141 (0.01640)	-0.1135 (0.01937)	<0.0001
	95% CI for LS MEAN	(-0.1488, -0.1064)	(-0.0470, 0.0188)	(-0.1523, -0.0746)	
Week 6	LS Mean (SEM)	-0.1180 (0.00820)	-0.0013 (0.01226)	-0.1167 (0.01455)	<0.0001
	95% CI for LS MEAN	(-0.1345, -0.1016)	(-0.0260, 0.0233)	(-0.1460, -0.0874)	
Week 8	LS Mean (SEM)	-0.1113 (0.00741)	-0.0116 (0.01080)	-0.0997 (0.01285)	<0.0001
	95% CI for LS MEAN	(-0.1262, -0.0965)	(-0.0333, 0.0101)	(-0.1255, -0.0739)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:53:36

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.4.1.7
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-0.1001 (0.00872)	0.0013 (0.01256)	-0.1015 (0.01512)	<0.0001
	95% CI for LS MEAN	(-0.1175, -0.0827)	(-0.0238, 0.0265)	(-0.1317, -0.0712)	
Week 12	LS Mean (SEM)	-0.0971 (0.00882)	-0.0072 (0.01305)	-0.0899 (0.01557)	<0.0001
	95% CI for LS MEAN	(-0.1147, -0.0795)	(-0.0333, 0.0189)	(-0.1210, -0.0588)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:53:36

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Table GERMANY.2.2.4.1.7
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.0710
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.4.1.8
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-0.0779 (0.01256)	-0.0206 (0.01721)	-0.0573 (0.01977)	0.0080
	95% CI for LS MEAN	(-0.1037, -0.0522)	(-0.0561, 0.0149)	(-0.0981, -0.0165)	
Week 2	LS Mean (SEM)	-0.1159 (0.01489)	-0.0131 (0.02147)	-0.1027 (0.02487)	0.0004
	95% CI for LS MEAN	(-0.1464, -0.0853)	(-0.0573, 0.0310)	(-0.1540, -0.0515)	
Week 3	LS Mean (SEM)	-0.1315 (0.01467)	-0.0157 (0.02106)	-0.1157 (0.02437)	0.0001
	95% CI for LS MEAN	(-0.1617, -0.1012)	(-0.0593, 0.0278)	(-0.1664, -0.0651)	
Week 4	LS Mean (SEM)	-0.1275 (0.01615)	-0.0167 (0.02339)	-0.1108 (0.02728)	0.0004
	95% CI for LS MEAN	(-0.1605, -0.0945)	(-0.0646, 0.0313)	(-0.1669, -0.0548)	
Week 6	LS Mean (SEM)	-0.1139 (0.01355)	-0.0173 (0.01960)	-0.0966 (0.02249)	0.0002
	95% CI for LS MEAN	(-0.1415, -0.0862)	(-0.0573, 0.0227)	(-0.1426, -0.0506)	
Week 8	LS Mean (SEM)	-0.0921 (0.01270)	-0.0128 (0.01775)	-0.0792 (0.02029)	0.0009
	95% CI for LS MEAN	(-0.1183, -0.0658)	(-0.0497, 0.0240)	(-0.1215, -0.0369)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:53:37

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.4.1.8
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-0.0924 (0.01304)	-0.0028 (0.01854)	-0.0897 (0.02143)	0.0003
	95% CI for LS MEAN	(-0.1192, -0.0656)	(-0.0409, 0.0353)	(-0.1338, -0.0455)	
Week 12	LS Mean (SEM)	-0.0891 (0.01521)	-0.0023 (0.02173)	-0.0868 (0.02526)	0.0021
	95% CI for LS MEAN	(-0.1203, -0.0579)	(-0.0470, 0.0424)	(-0.1389, -0.0347)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:53:37

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Table GERMANY.2.2.4.1.8
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-0.0616 (0.00747)	-0.0100 (0.01324)	-0.0515 (0.01524)	0.0023
	95% CI for LS MEAN	(-0.0770, -0.0461)	(-0.0372, 0.0171)	(-0.0828, -0.0202)	
Week 2	LS Mean (SEM)	-0.1171 (0.01069)	-0.0024 (0.01879)	-0.1146 (0.02156)	<0.0001
	95% CI for LS MEAN	(-0.1389, -0.0953)	(-0.0407, 0.0358)	(-0.1586, -0.0707)	
Week 3	LS Mean (SEM)	-0.1247 (0.01123)	-0.0091 (0.01935)	-0.1156 (0.02229)	<0.0001
	95% CI for LS MEAN	(-0.1477, -0.1017)	(-0.0488, 0.0305)	(-0.1612, -0.0699)	
Week 4	LS Mean (SEM)	-0.1160 (0.01365)	-0.0037 (0.02294)	-0.1123 (0.02662)	0.0002
	95% CI for LS MEAN	(-0.1439, -0.0881)	(-0.0506, 0.0432)	(-0.1667, -0.0579)	
Week 6	LS Mean (SEM)	-0.1140 (0.01210)	0.0075 (0.02030)	-0.1215 (0.02352)	<0.0001
	95% CI for LS MEAN	(-0.1389, -0.0892)	(-0.0342, 0.0492)	(-0.1699, -0.0732)	
Week 8	LS Mean (SEM)	-0.1031 (0.01046)	0.0067 (0.01681)	-0.1098 (0.01973)	<0.0001
	95% CI for LS MEAN	(-0.1246, -0.0815)	(-0.0279, 0.0413)	(-0.1504, -0.0692)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:53:37

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.4.1.8
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-0.0743 (0.01129)	0.0038 (0.01575)	-0.0781 (0.01937)	0.0005
	95% CI for LS MEAN	(-0.0975, -0.0510)	(-0.0289, 0.0365)	(-0.1182, -0.0380)	
Week 12	LS Mean (SEM)	-0.0821 (0.01224)	0.0048 (0.01860)	-0.0869 (0.02217)	0.0005
	95% CI for LS MEAN	(-0.1070, -0.0572)	(-0.0333, 0.0428)	(-0.1322, -0.0416)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:53:37

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Table GERMANY.2.2.4.1.8
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 1	LS Mean (SEM)	-0.1227 (0.03312)	0.0133 (0.03705)	-0.1359 (0.04354)	0.0099
	95% CI for LS MEAN	(-0.1983, -0.0471)	(-0.0694, 0.0959)	(-0.2320, -0.0399)	
Week 2	LS Mean (SEM)	-0.1769 (0.03252)	-0.0226 (0.03609)	-0.1543 (0.04228)	0.0044
	95% CI for LS MEAN	(-0.2519, -0.1020)	(-0.1038, 0.0586)	(-0.2485, -0.0602)	
Week 3	LS Mean (SEM)	-0.1700 (0.03252)	-0.0452 (0.03609)	-0.1248 (0.04228)	0.0144
	95% CI for LS MEAN	(-0.2449, -0.0951)	(-0.1264, 0.0359)	(-0.2189, -0.0306)	
Week 4	LS Mean (SEM)	-0.1615 (0.03276)	-0.0456 (0.03692)	-0.1159 (0.04314)	0.0216
	95% CI for LS MEAN	(-0.2366, -0.0863)	(-0.1278, 0.0367)	(-0.2112, -0.0206)	
Week 6	LS Mean (SEM)	-0.1375 (0.03281)	-0.0285 (0.03609)	-0.1090 (0.04245)	0.0277
	95% CI for LS MEAN	(-0.2127, -0.0623)	(-0.1097, 0.0527)	(-0.2033, -0.0146)	
Week 8	LS Mean (SEM)	-0.1696 (0.03367)	-0.0629 (0.03609)	-0.1066 (0.04328)	0.0322
	95% CI for LS MEAN	(-0.2462, -0.0930)	(-0.1441, 0.0182)	(-0.2023, -0.0109)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:53:37

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.4.1.8
Change from Baseline in Absolute Reticulocyte Count (10¹²/L) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 10	LS Mean (SEM)	-0.1529 (0.03322)	-0.0491 (0.03609)	-0.1038 (0.04290)	0.0351
	95% CI for LS MEAN	(-0.2287, -0.0771)	(-0.1303, 0.0321)	(-0.1989, -0.0088)	
Week 12	LS Mean (SEM)	-0.1520 (0.03276)	-0.0484 (0.03697)	-0.1036 (0.04343)	0.0366
	95% CI for LS MEAN	(-0.2271, -0.0768)	(-0.1308, 0.0341)	(-0.1995, -0.0078)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Run Date: 2024-04-26T15:53:37

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Table GERMANY.2.2.4.1.8
Change from Baseline in Absolute Reticulocyte Count ($10^{12}/L$) During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.1132
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on a MMRM model which includes categorical variables for treatment, study visit, and study visit by treatment group interaction as well as continuous covariates baseline absolute reticulocyte count, and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADLB

Data cutoff date of 31Mar2023

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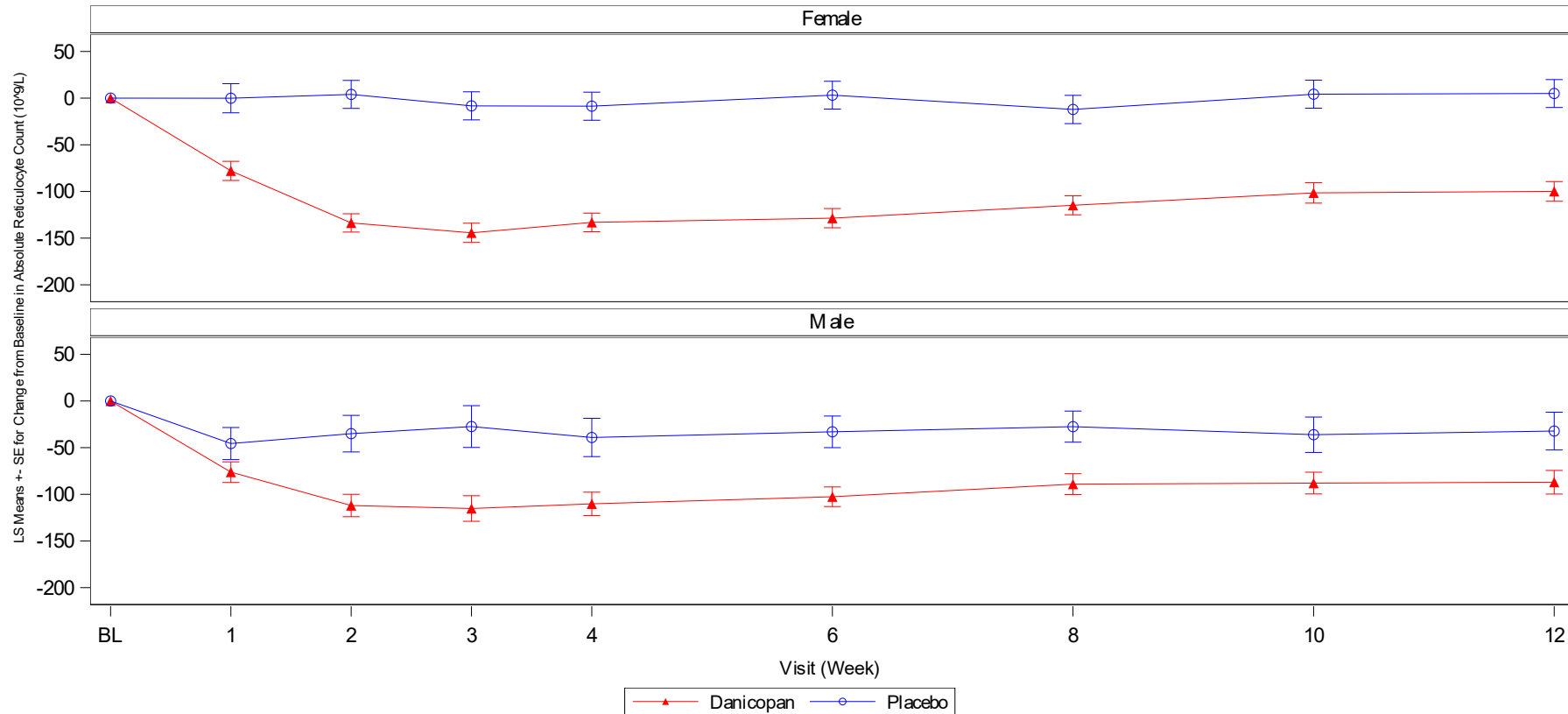
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Figure GERMANY.2.2.2.1.2
Plots of LS Means \pm SE for Change from Baseline in Absolute Reticulocyte Count ($10^9/L$)
During 12-Week of Treatment Period 1 by Sex
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).
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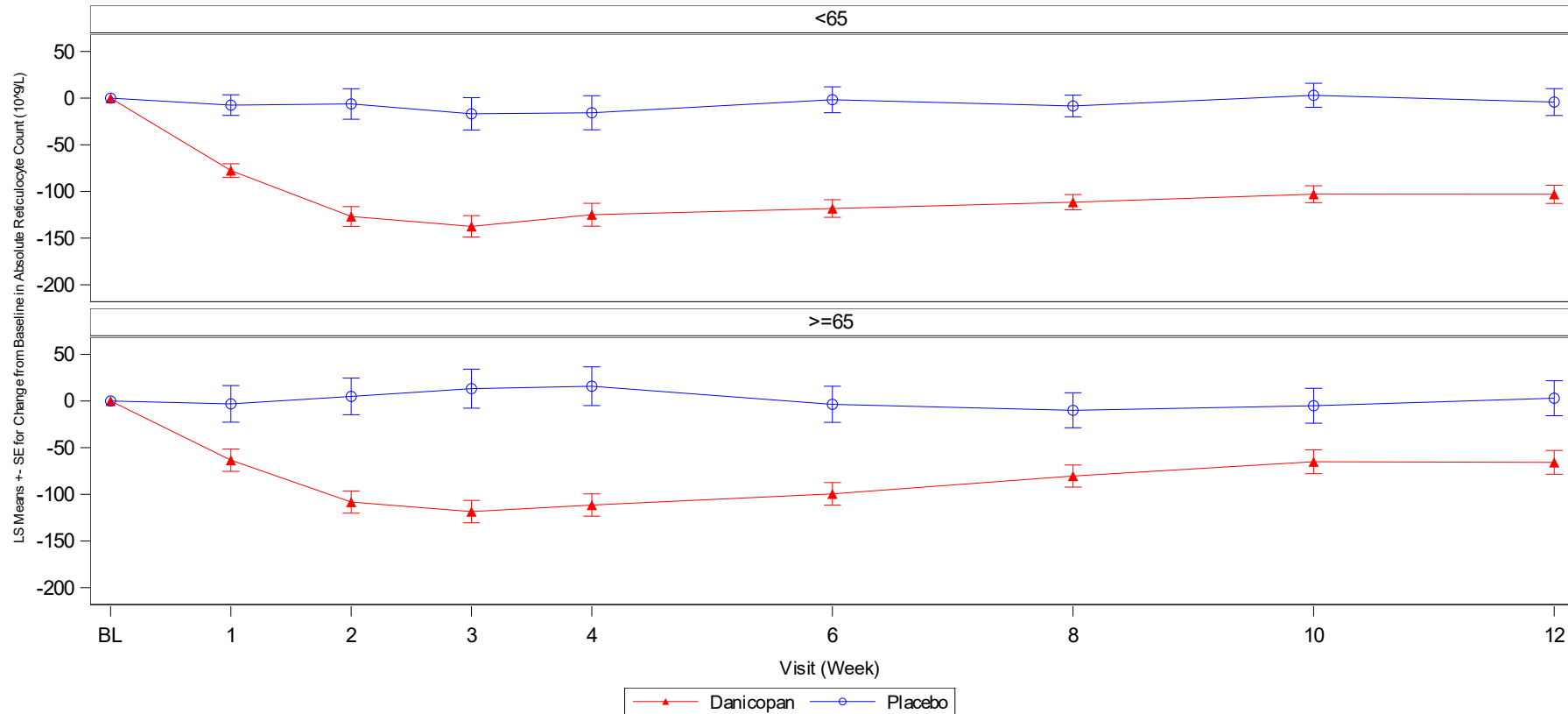
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Figure GERMANY.2.2.2.1.3
Plots of LS Means \pm SE for Change from Baseline in Absolute Reticulocyte Count ($10^9/L$) During 12-Week of Treatment Period 1 by Age at Informed Consent (< 65 and \geq 65 Years) Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).
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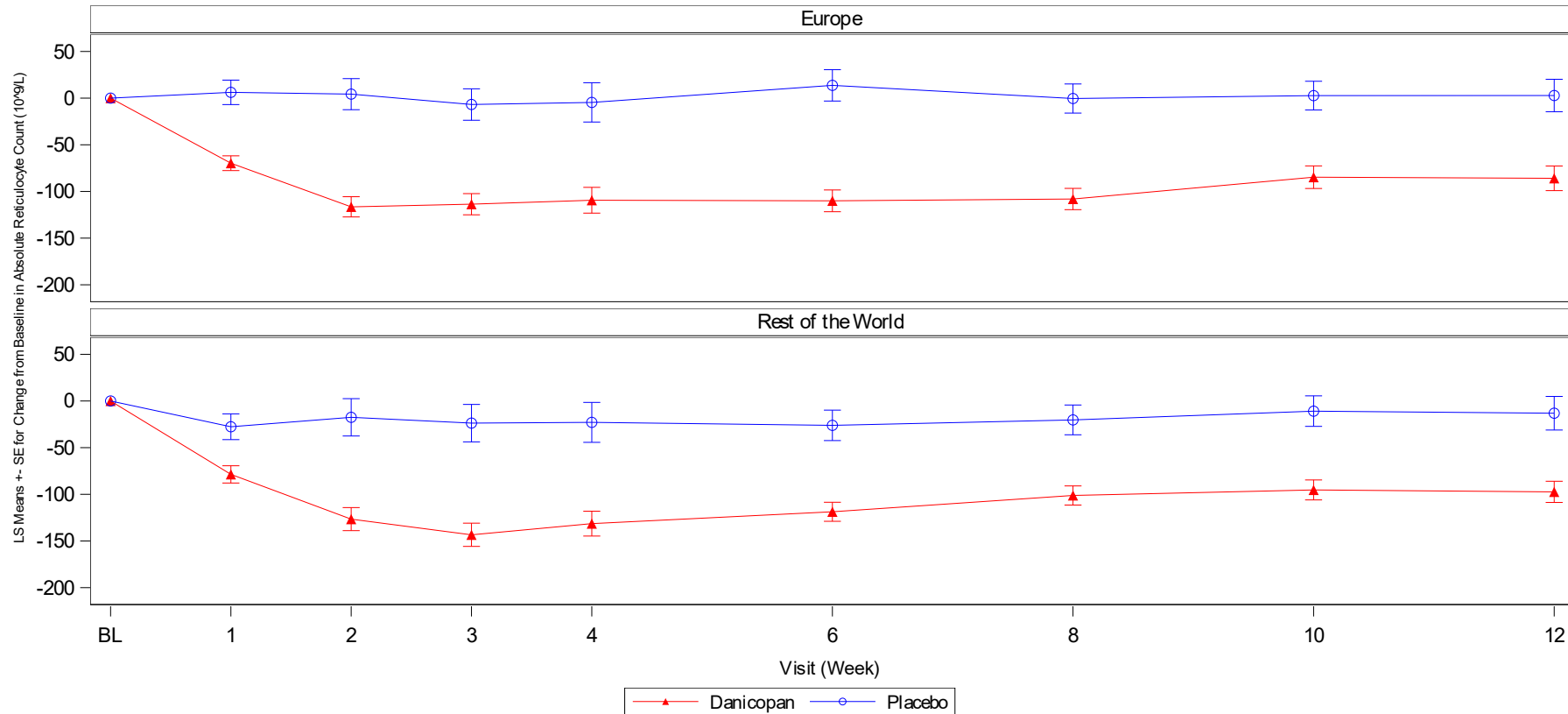
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Figure GERMANY.2.2.2.1.4
Plots of LS Means \pm SE for Change from Baseline in Absolute Reticulocyte Count ($10^9/L$)
During 12-Week of Treatment Period 1 by Region
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).
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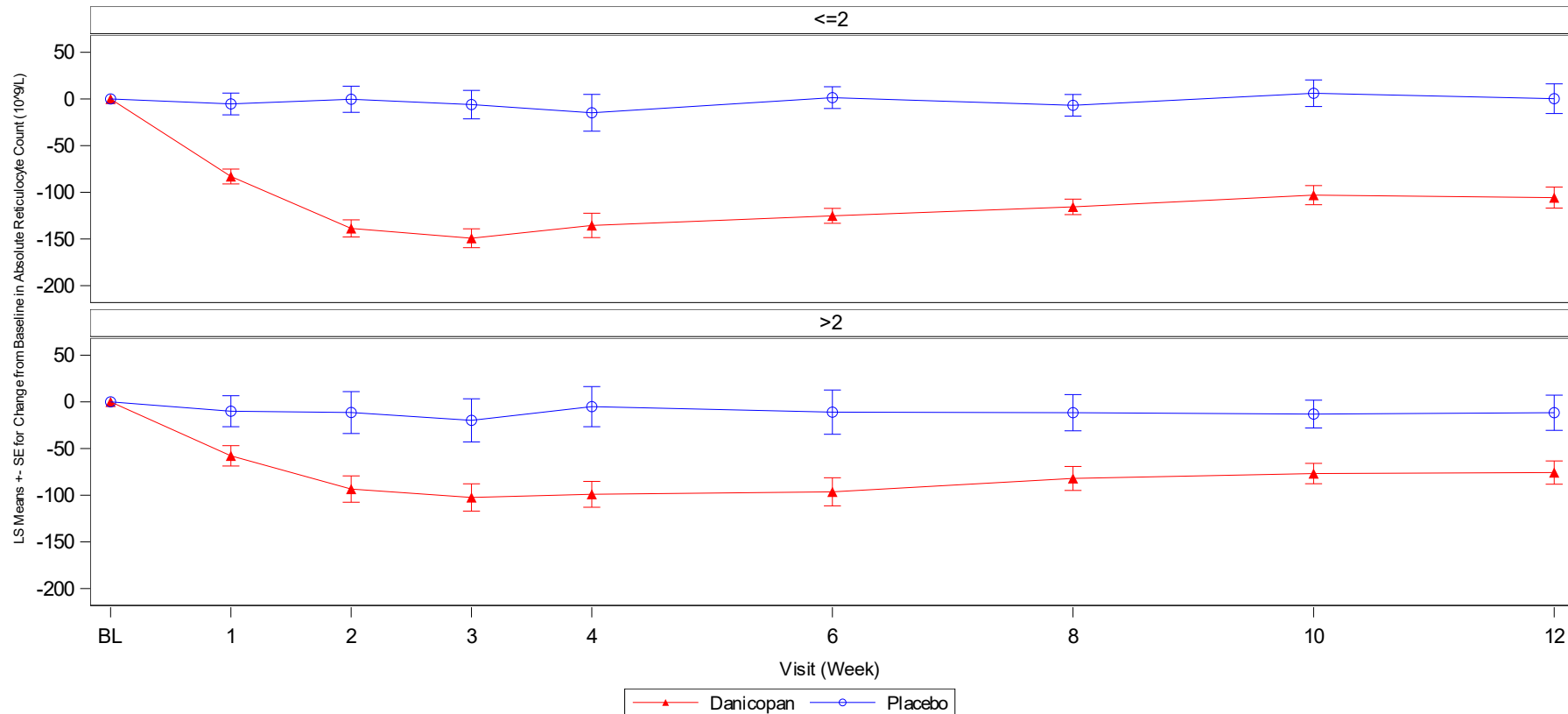
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Figure GERMANY.2.2.2.1.5
Plots of LS Means \pm SE for Change from Baseline in Absolute Reticulocyte Count ($10^9/L$) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or \leq 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).
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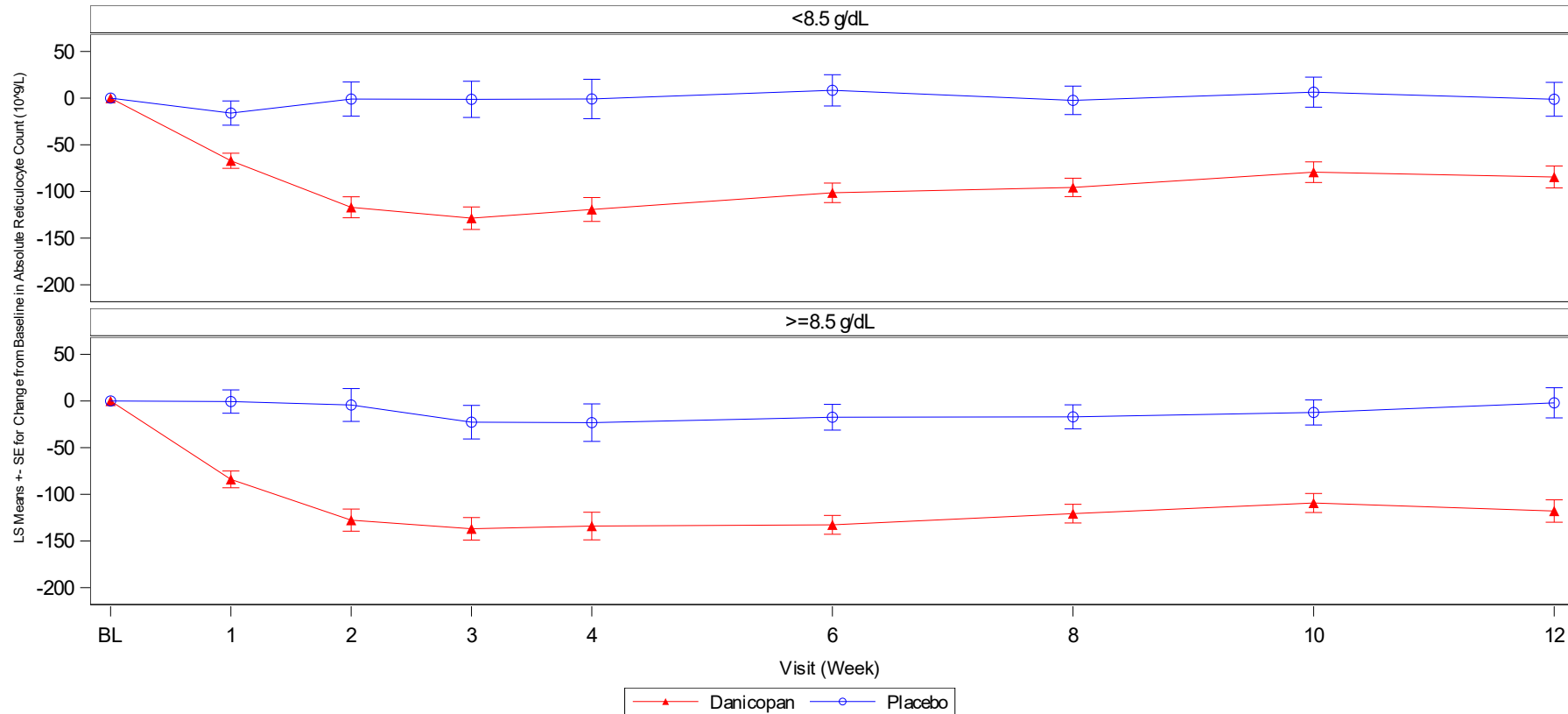
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Figure GERMANY.2.2.2.1.6

Plots of LS Means \pm SE for Change from Baseline in Absolute Reticulocyte Count ($10^9/L$) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).
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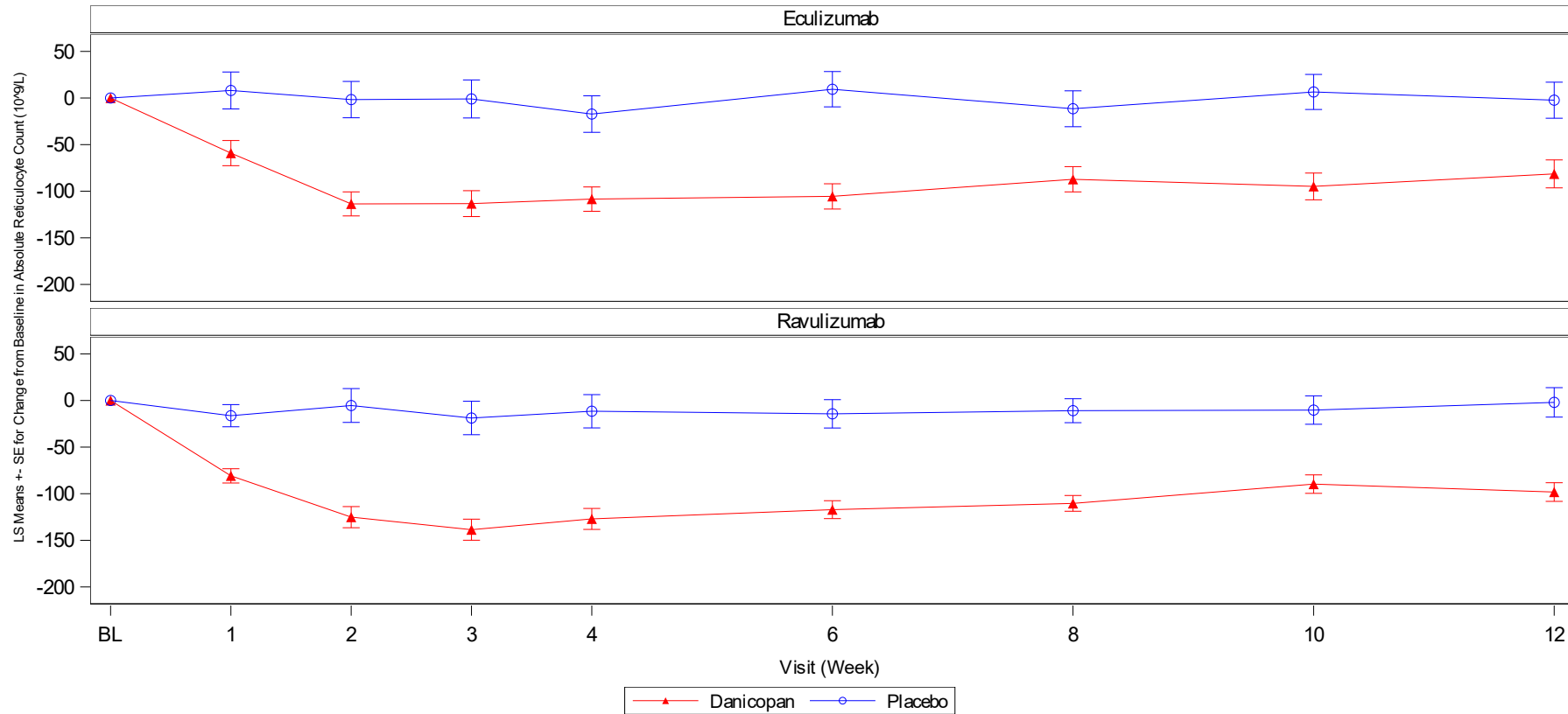
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Figure GERMANY.2.2.2.1.7
Plots of LS Means \pm SE for Change from Baseline in Absolute Reticulocyte Count ($10^9/L$) During 12-Week of Treatment Period 1 by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).
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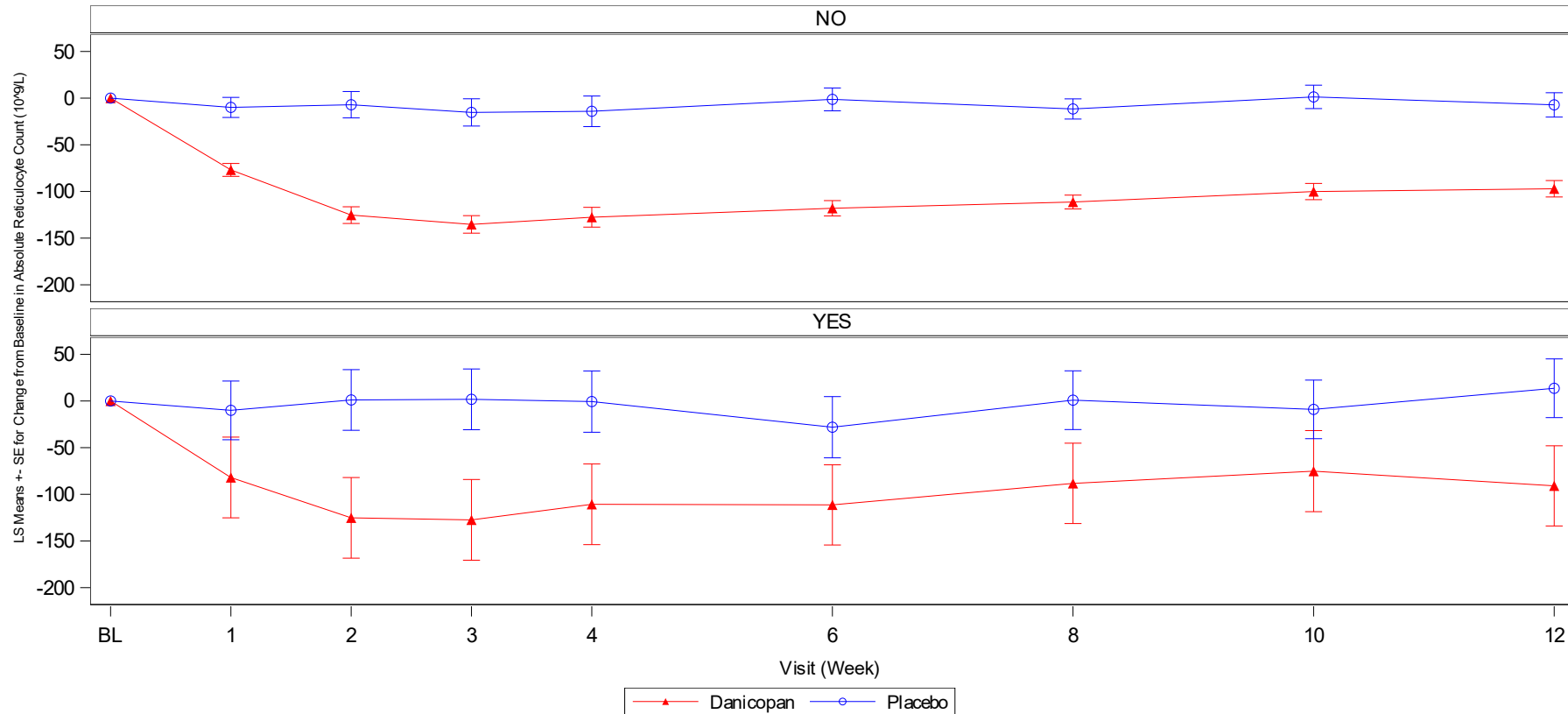
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Figure GERMANY.2.2.2.1.8

Plots of LS Means \pm SE for Change from Baseline in Absolute Reticulocyte Count ($10^9/L$) During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).
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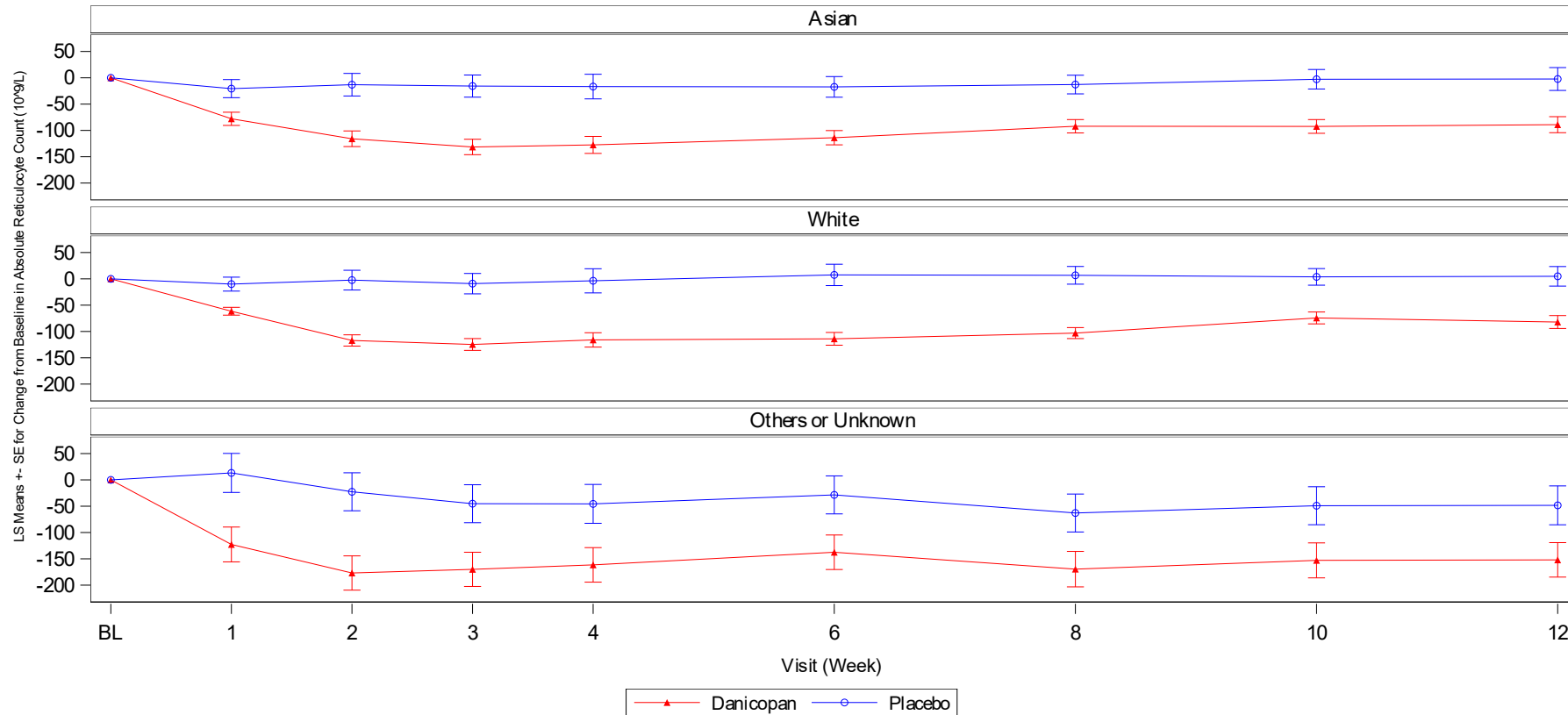
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Figure GERMANY.2.2.2.1.9
Plots of LS Means \pm SE for Change from Baseline in Absolute Reticulocyte Count ($10^9/L$)
During 12-Week of Treatment Period 1 by Race
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).
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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom

31.03.2023 1 Morbidität

1.5 FACIT-Fatigue-Skala

1.5.1 Rücklaufquoten der FACIT-Fatigue-Skala

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Table GERMANY.2.6.3.2
FACIT Fatigue Scores Compliance Rate During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit	Statistics	D->D (N=57)	P->D (N=26)
Baseline	Expected Patients Per Visit	57	26
	Patients Per Visit	57	26
	Patients with at Least One Completed Question, n (%)	56 (98.2)	25 (96.2)
	Patients with Fully Completed Questionnaire, n (%)	55 (96.5)	25 (96.2)
Week 2	Expected Patients Per Visit	57	26
	Patients Per Visit	57	26
	Patients with at Least One Completed Question, n (%)	56 (98.2)	25 (96.2)
	Patients with Fully Completed Questionnaire, n (%)	55 (96.5)	25 (96.2)
Week 4	Expected Patients Per Visit	57	25
	Patients Per Visit	57	25
	Patients with at Least One Completed Question, n (%)	57 (100)	25 (100)
	Patients with Fully Completed Questionnaire, n (%)	57 (100)	25 (100)
Week 8	Expected Patients Per Visit	57	25
	Patients Per Visit	57	25
	Patients with at Least One Completed Question, n (%)	45 (78.9)	19 (76.0)
	Patients with Fully Completed Questionnaire, n (%)	44 (77.2)	18 (72.0)
Week 12	Expected Patients Per Visit	55	25
	Patients Per Visit	54	25
	Patients with at Least One Completed Question, n (%)	52 (94.5)	25 (100)
	Patients with Fully Completed Questionnaire, n (%)	51 (92.7)	25 (100)

Percentages are based on the total number of expected patients in each group.

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.2
FACIT Fatigue Scores Compliance Rate During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit	Statistics	D->D (N=57)	P->D (N=26)
Week 14	Expected Patients Per Visit	55	24
	Patients Per Visit	55	24
	Patients with at Least One Completed Question, n (%)	52 (94.5)	24 (100)
	Patients with Fully Completed Questionnaire, n (%)	52 (94.5)	23 (95.8)
Week 16	Expected Patients Per Visit	55	24
	Patients Per Visit	55	24
	Patients with at Least One Completed Question, n (%)	53 (96.4)	23 (95.8)
	Patients with Fully Completed Questionnaire, n (%)	53 (96.4)	23 (95.8)
Week 20	Expected Patients Per Visit	55	24
	Patients Per Visit	53	24
	Patients with at Least One Completed Question, n (%)	47 (85.5)	20 (83.3)
	Patients with Fully Completed Questionnaire, n (%)	47 (85.5)	20 (83.3)
Week 24	Expected Patients Per Visit	55	24
	Patients Per Visit	55	24
	Patients with at Least One Completed Question, n (%)	50 (90.9)	24 (100)
	Patients with Fully Completed Questionnaire, n (%)	49 (89.1)	23 (95.8)
Week 40	Expected Patients Per Visit	46	22
	Patients Per Visit	46	22
	Patients with at Least One Completed Question, n (%)	45 (97.8)	21 (95.5)
	Patients with Fully Completed Questionnaire, n (%)	45 (97.8)	21 (95.5)

Percentages are based on the total number of expected patients in each group.

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.6.3.2
FACIT Fatigue Scores Compliance Rate During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit	Statistics	D->D (N=57)	P->D (N=26)
Week 56	Expected Patients Per Visit	36	18
	Patients Per Visit	36	18
	Patients with at Least One Completed Question, n (%)	36 (100)	17 (94.4)
	Patients with Fully Completed Questionnaire, n (%)	36 (100)	17 (94.4)
Week 72	Expected Patients Per Visit	21	12
	Patients Per Visit	21	12
	Patients with at Least One Completed Question, n (%)	21 (100)	12 (100)
	Patients with Fully Completed Questionnaire, n (%)	21 (100)	12 (100)
Week 88	Expected Patients Per Visit	14	8
	Patients Per Visit	14	8
	Patients with at Least One Completed Question, n (%)	14 (100)	8 (100)
	Patients with Fully Completed Questionnaire, n (%)	14 (100)	8 (100)
Week 104	Expected Patients Per Visit	4	2
	Patients Per Visit	4	2
	Patients with at Least One Completed Question, n (%)	4 (100)	2 (100)
	Patients with Fully Completed Questionnaire, n (%)	4 (100)	2 (100)

Percentages are based on the total number of expected patients in each group.

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Source: ADaM.ADSL, ADaM.ADQS

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.5 FACIT-Fatigue-Skala

1.5.2 Veränderung der FACIT-Fatigue-Skala gegenüber Baseline

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Table GERMANY.2.6.3.1
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	56		25		81	
Mean (SD)	34.0238 (11.26514)		32.8400 (10.93801)		33.6584 (11.11040)	
Median	36.00		33.00		35.00	
Min, Max	6.0, 52.0		12.0, 52.0		6.0, 52.0	
Week 2						
n	56	55	25	24		
Mean (SD)	39.3542 (10.11720)	5.3727 (7.58342)	34.2000 (9.67385)	1.9167 (8.29309)		
Median	42.50	5.00	37.00	0.00		
Min, Max	9.0, 52.0	-8.0, 33.0	15.0, 52.0	-11.0, 25.0		
Week 4						
n	57	56	25	25		
Mean (SD)	40.8070 (9.49518)	6.9226 (9.25961)	35.7600 (10.26840)	2.9200 (9.38048)		
Median	44.00	6.00	37.00	2.00		
Min, Max	9.0, 52.0	-14.0, 36.0	16.0, 52.0	-13.0, 25.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	45	44	19	19		
Mean (SD)	41.4370 (7.95182)	7.5758 (8.54933)	35.0658 (9.81348)	1.8026 (11.58860)		
Median	42.00	6.00	35.00	4.00		
Min, Max	17.0, 52.0	-8.0, 36.0	16.3, 52.0	-20.0, 23.0		
Week 12						
n	52	51	25	25		
Mean (SD)	42.1399 (7.69362)	8.2537 (7.66088)	35.4000 (10.65364)	2.5600 (9.37230)		
Median	42.50	6.83	38.00	1.00		
Min, Max	20.0, 52.0	-3.0, 26.0	10.0, 52.0	-10.0, 26.0		
Week 14						
n	52	51	24	24		
Mean (SD)	42.9808 (7.37641)	8.6797 (8.42673)	37.5729 (10.46565)	3.8646 (9.30550)		
Median	44.00	7.00	41.00	4.50		
Min, Max	19.0, 52.0	-3.0, 35.0	16.0, 52.0	-15.0, 21.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.3.1
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	53	52	23	23		
Mean (SD)	41.7170 (8.67219)	7.6474 (9.59125)	40.2609 (9.60217)	6.5217 (8.26201)		
Median	43.00	6.50	43.00	6.00		
Min, Max	4.0, 52.0	-13.2, 35.0	20.0, 52.0	-12.0, 20.0		
Week 20						
n	47	46	20	20		
Mean (SD)	40.7447 (10.19051)	5.3188 (8.36356)	38.2000 (11.89250)	3.5000 (11.86991)		
Median	43.00	4.00	41.00	4.00		
Min, Max	4.0, 52.0	-13.0, 36.0	9.0, 52.0	-24.0, 19.0		
Week 24						
n	50	49	24	24		
Mean (SD)	40.9400 (9.73403)	6.7891 (8.72919)	39.3750 (11.11134)	5.6667 (9.68040)		
Median	43.00	5.00	42.50	5.50		
Min, Max	7.0, 52.0	-10.0, 36.0	13.0, 52.0	-13.0, 19.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	45	45	21	21	66	66
Mean (SD)	41.0889 (9.84614)	6.4185 (8.23446)	34.9524 (12.43976)	0.4286 (10.70314)	39.1364 (11.02429)	4.5126 (9.43671)
Median	45.00	5.00	36.00	0.00	42.50	4.42
Min, Max	10.0, 52.0	-9.0, 27.0	9.0, 52.0	-25.0, 15.0	9.0, 52.0	-25.0, 27.0
Week 56						
n	36	36	17	17	53	53
Mean (SD)	39.2500 (9.96673)	5.6065 (8.12718)	40.7059 (5.33578)	5.2353 (10.70940)	39.7170 (8.72304)	5.4874 (8.93183)
Median	40.50	3.50	42.00	2.00	41.00	3.00
Min, Max	17.0, 52.0	-6.0, 31.0	27.0, 50.0	-10.0, 25.0	17.0, 52.0	-10.0, 31.0
Week 72						
n	21	21	12	12	33	33
Mean (SD)	41.5238 (10.33740)	6.8095 (8.64071)	36.6667 (12.11560)	2.3333 (10.03026)	39.7576 (11.08499)	5.1818 (9.27515)
Median	45.00	6.00	41.50	1.50	43.00	6.00
Min, Max	17.0, 52.0	-8.0, 31.0	8.0, 49.0	-14.0, 22.0	8.0, 52.0	-14.0, 31.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit/Statistics	D->D (N = 57)		P->D (N = 26)		Total (N = 83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	45.6429 (6.00778)	8.3571 (7.06679)	37.1250 (11.75266)	0.6250 (11.72223)	42.5455 (9.27222)	5.5455 (9.55050)
Median	48.00	7.50	40.50	-2.50	45.00	6.00
Min, Max	32.0, 52.0	0.0, 22.0	14.0, 52.0	-12.0, 23.0	14.0, 52.0	-12.0, 23.0
Week 104						
n	4	4	2	2	6	6
Mean (SD)	40.5000 (8.66025)	2.5000 (13.82027)	39.0000 (1.41421)	8.0000 (18.38478)	40.0000 (6.78233)	4.3333 (13.79372)
Median	39.50	0.50	39.00	8.00	39.00	0.50
Min, Max	32.0, 51.0	-12.0, 21.0	38.0, 40.0	-5.0, 21.0	32.0, 51.0	-12.0, 21.0

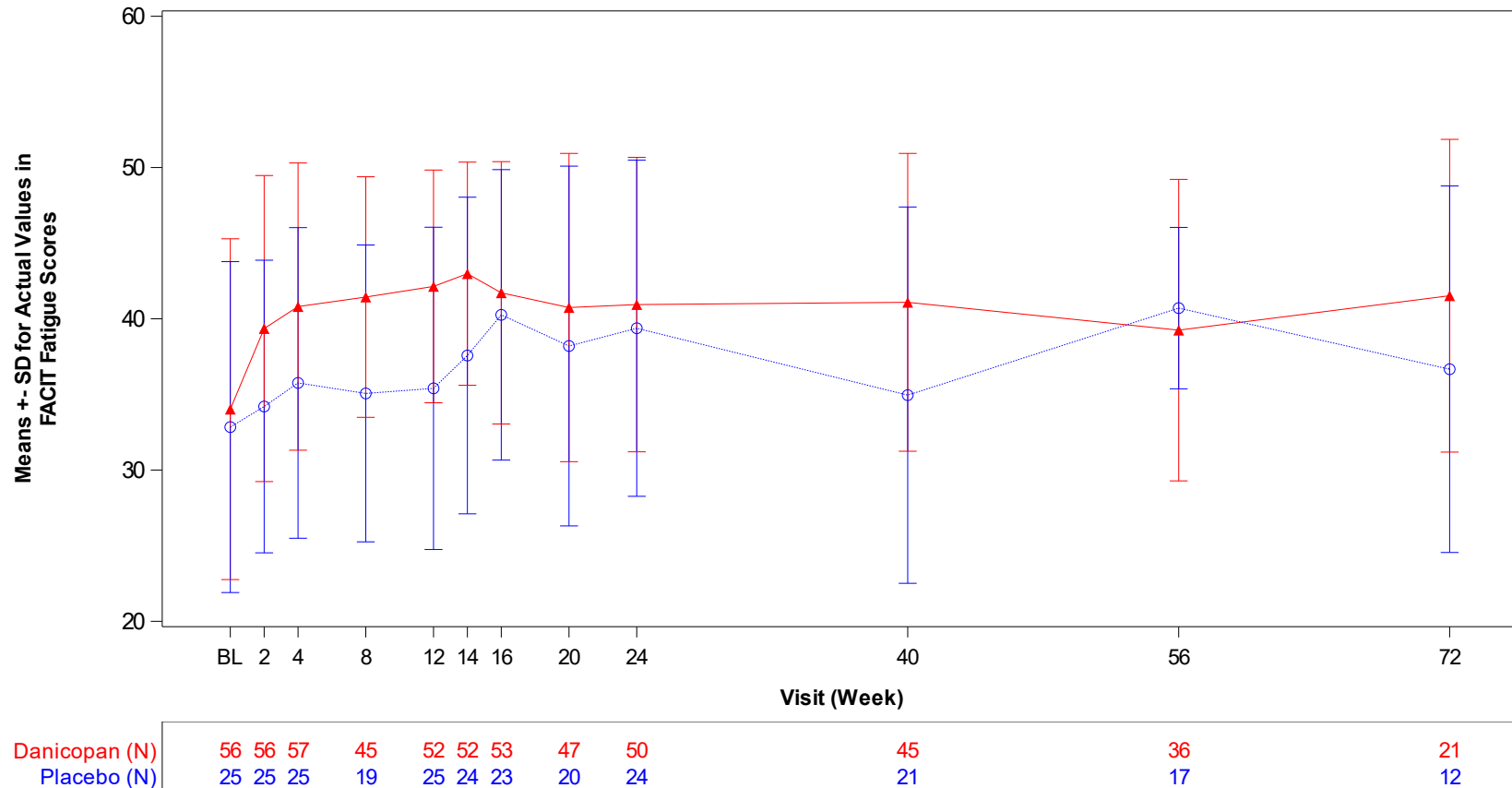
Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Source: ADaM.ADSL, ADaM.ADQS
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Figure GERMANY.2.2.1.3
Plots of Mean \pm SD for Actual Values in FACIT Fatigue Scores During 72 Weeks
Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Source: ADaM.ADQS

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Table GERMANY.2.2.3.1
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Visit	Statistics	Danicopan (N = 56)	Placebo (N = 25)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.66 (0.955)	1.19 (1.426)	4.48 (1.689)	0.0098
	95% CI for LS MEAN	(3.76, 7.57)	(-1.65, 4.02)	(1.11, 7.84)	
Week 4	LS Mean (SEM)	7.13 (1.062)	2.65 (1.572)	4.48 (1.874)	0.0194
	95% CI for LS MEAN	(5.02, 9.24)	(-0.48, 5.78)	(0.74, 8.21)	
Week 8	LS Mean (SEM)	7.44 (1.142)	-0.41 (1.703)	7.85 (2.028)	0.0003
	95% CI for LS MEAN	(5.16, 9.72)	(-3.81, 2.99)	(3.80, 11.90)	
Week 12	LS Mean (SEM)	8.00 (0.917)	2.29 (1.321)	5.71 (1.581)	0.0006
	95% CI for LS MEAN	(6.18, 9.83)	(-0.34, 4.93)	(2.56, 8.86)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). An Unstructured covariance matrix was used. FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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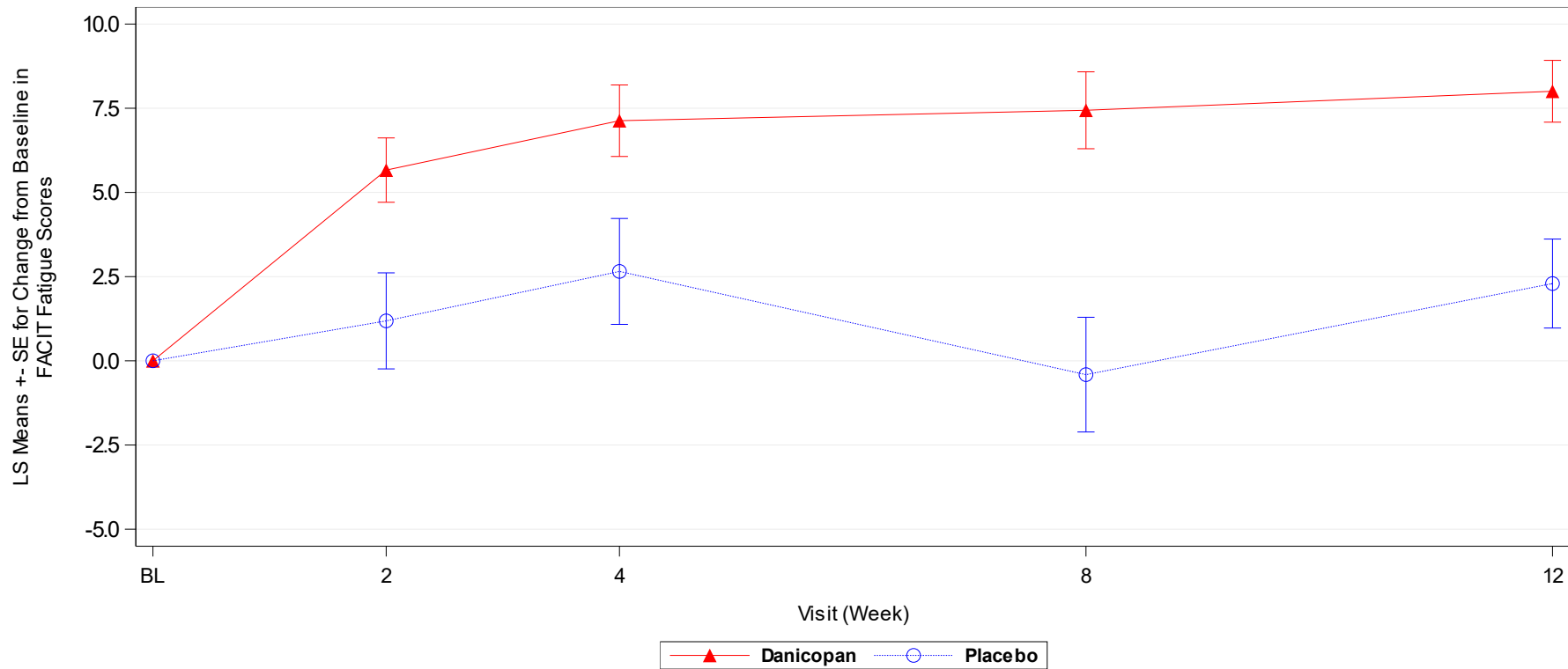
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Figure GERMANY.2.2.1.1
Plots of LS Means \pm SE for Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).
FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.5 FACIT-Fatigue-Skala

1.5.2 Veränderung der FACIT-Fatigue-Skala gegenüber Baseline

1.5.2.1 Subgruppenanalysen – Veränderung der FACIT-Fatigue-Skala gegenüber Baseline

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Table GERMANY.2.6.3.1.1
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	36.32 (11.137)		39.11 (8.536)		37.13 (10.388)	
Median	38.50		41.00		39.00	
Min, Max	9.0, 51.0		26.0, 52.0		9.0, 52.0	
Week 2						
n	23	22	8	8		
Mean (SD)	41.21 (9.039)	5.17 (8.951)	40.50 (6.547)	2.25 (5.471)		
Median	43.00	4.00	38.00	3.00		
Min, Max	9.0, 51.0	-7.0, 33.0	33.0, 52.0	-8.0, 8.0		
Week 4						
n	23	22	9	9		
Mean (SD)	42.30 (8.583)	6.41 (9.490)	41.00 (7.550)	1.89 (8.923)		
Median	45.00	4.50	40.00	3.00		
Min, Max	14.0, 52.0	-4.0, 36.0	29.0, 52.0	-13.0, 13.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1.1
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	19	18	7	7		
Mean (SD)	42.25 (8.025)	5.43 (4.403)	42.71 (5.736)	2.71 (8.015)		
Median	43.00	5.50	42.00	4.00		
Min, Max	21.7, 52.0	0.0, 15.0	35.0, 52.0	-10.0, 16.0		
Week 12						
n	22	21	9	9		
Mean (SD)	42.55 (8.134)	6.71 (6.929)	40.44 (9.774)	1.33 (8.396)		
Median	44.00	6.00	42.00	1.00		
Min, Max	22.0, 52.0	-3.0, 26.0	19.0, 52.0	-10.0, 16.0		
Week 14						
n	22	21	9	9		
Mean (SD)	44.09 (7.023)	8.10 (8.831)	41.67 (8.832)	2.56 (7.401)		
Median	44.50	7.00	44.00	3.00		
Min, Max	23.0, 52.0	-3.0, 35.0	23.0, 52.0	-9.0, 18.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1.1
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	22	21	8	8		
Mean (SD)	44.00 (5.888)	8.29 (8.673)	45.50 (6.279)	5.63 (8.501)		
Median	44.00	6.00	47.00	7.50		
Min, Max	33.0, 52.0	-2.0, 32.0	36.0, 52.0	-9.0, 19.0		
Week 20						
n	20	19	9	9		
Mean (SD)	44.30 (6.736)	7.05 (9.583)	43.44 (8.791)	4.33 (12.845)		
Median	45.00	6.00	45.00	9.00		
Min, Max	30.0, 52.0	-3.0, 36.0	25.0, 52.0	-21.0, 19.0		
Week 24						
n	21	20	9	9		
Mean (SD)	42.67 (9.216)	6.60 (9.735)	44.22 (7.155)	5.11 (10.810)		
Median	44.00	4.00	45.00	4.00		
Min, Max	19.0, 52.0	-7.0, 36.0	34.0, 52.0	-10.0, 19.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
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Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.6.3.1.1
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	8	8	29	29
Mean (SD)	42.76 (8.514)	7.05 (8.435)	42.38 (9.985)	4.00 (12.547)	42.66 (8.760)	6.21 (9.597)
Median	45.00	5.00	43.50	8.00	45.00	5.00
Min, Max	19.0, 52.0	-3.0, 27.0	21.0, 52.0	-25.0, 13.0	19.0, 52.0	-25.0, 27.0
Week 56						
n	17	17	5	5	22	22
Mean (SD)	41.35 (9.546)	6.94 (10.047)	44.60 (4.980)	3.80 (10.710)	42.09 (8.723)	6.23 (10.028)
Median	42.00	4.00	44.00	1.00	44.00	3.50
Min, Max	17.0, 52.0	-5.0, 31.0	37.0, 50.0	-9.0, 18.0	17.0, 52.0	-9.0, 31.0
Week 72						
n	8	8	2	2	10	10
Mean (SD)	44.63 (3.852)	11.00 (10.967)	45.50 (4.950)	1.00 (7.071)	44.80 (3.795)	9.00 (10.812)
Median	45.00	8.00	45.50	1.00	45.00	6.50
Min, Max	40.0, 52.0	-1.0, 31.0	42.0, 49.0	-4.0, 6.0	40.0, 52.0	-4.0, 31.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1.1
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Male

Visit/Statistics	D->D (N = 23)		P->D (N = 9)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	2	2	8	8
Mean (SD)	46.33 (2.338)	10.50 (7.503)	43.00 (12.728)	-1.50 (14.849)	45.50 (5.425)	7.50 (10.128)
Median	47.00	9.50	43.00	-1.50	47.00	8.50
Min, Max	42.0, 48.0	0.0, 22.0	34.0, 52.0	-12.0, 9.0	34.0, 52.0	-12.0, 22.0
Week 104						
n	1	1	0	0	1	1
Mean (SD)	44.00 (NA)	-2.00 (NA)			44.00 (NA)	-2.00 (NA)
Median	44.00	-2.00			44.00	-2.00
Min, Max	44.0, 44.0	-2.0, -2.0			44.0, 44.0	-2.0, -2.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1.1
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		16		50	
Mean (SD)	32.54 (11.260)		29.31 (10.756)		31.51 (11.096)	
Median	34.50		29.50		32.50	
Min, Max	6.0, 52.0		12.0, 45.0		6.0, 52.0	
Week 2						
n	33	33	17	16		
Mean (SD)	38.06 (10.750)	5.51 (6.665)	31.24 (9.615)	1.75 (9.560)		
Median	42.00	7.00	34.00	0.00		
Min, Max	11.0, 52.0	-8.0, 25.0	15.0, 49.0	-11.0, 25.0		
Week 4						
n	34	34	16	16		
Mean (SD)	39.79 (10.063)	7.25 (9.236)	32.81 (10.610)	3.50 (9.866)		
Median	43.00	7.42	34.00	1.50		
Min, Max	9.0, 52.0	-14.0, 27.0	16.0, 49.0	-12.0, 25.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.6.3.1.1
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	26	26	12	12		
Mean (SD)	40.85 (8.003)	9.06 (10.339)	30.60 (8.983)	1.27 (13.560)		
Median	41.00	7.92	29.50	1.00		
Min, Max	17.0, 52.0	-8.0, 36.0	16.3, 47.0	-20.0, 23.0		
Week 12						
n	30	30	16	16		
Mean (SD)	41.84 (7.481)	9.33 (8.072)	32.56 (10.334)	3.25 (10.076)		
Median	41.50	7.50	34.50	2.00		
Min, Max	20.0, 52.0	-2.0, 24.0	10.0, 44.0	-9.0, 26.0		
Week 14						
n	30	30	15	15		
Mean (SD)	42.17 (7.639)	9.09 (8.260)	35.12 (10.869)	4.65 (10.450)		
Median	42.00	7.92	40.00	6.00		
Min, Max	19.0, 52.0	-2.0, 34.0	16.0, 48.0	-15.0, 21.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.1
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	31	31	15	15		
Mean (SD)	40.10 (9.978)	7.22 (10.284)	37.47 (10.056)	7.00 (8.392)		
Median	41.00	7.00	42.00	5.00		
Min, Max	4.0, 52.0	-13.2, 35.0	20.0, 51.0	-12.0, 20.0		
Week 20						
n	27	27	11	11		
Mean (SD)	38.11 (11.560)	4.10 (7.331)	33.91 (12.724)	2.82 (11.600)		
Median	40.00	3.00	39.00	3.00		
Min, Max	4.0, 52.0	-13.0, 23.0	9.0, 49.0	-24.0, 18.0		
Week 24						
n	29	29	15	15		
Mean (SD)	39.69 (10.064)	6.92 (8.141)	36.47 (12.223)	6.00 (9.320)		
Median	41.00	5.00	40.00	7.00		
Min, Max	7.0, 52.0	-10.0, 24.0	13.0, 52.0	-13.0, 17.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.1
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	24	24	13	13	37	37
Mean (SD)	39.63 (10.846)	5.87 (8.196)	30.38 (11.850)	-1.77 (9.239)	36.38 (11.915)	3.18 (9.221)
Median	41.00	5.92	33.00	-3.00	36.00	3.00
Min, Max	10.0, 52.0	-9.0, 25.0	9.0, 52.0	-15.0, 15.0	9.0, 52.0	-15.0, 25.0
Week 56						
n	19	19	12	12	31	31
Mean (SD)	37.37 (10.210)	4.41 (5.960)	39.08 (4.757)	5.83 (11.126)	38.03 (8.460)	4.96 (8.198)
Median	38.00	3.00	40.50	3.50	39.00	3.00
Min, Max	20.0, 52.0	-6.0, 16.0	27.0, 44.0	-10.0, 25.0	20.0, 52.0	-10.0, 25.0
Week 72						
n	13	13	10	10	23	23
Mean (SD)	39.62 (12.613)	4.23 (5.946)	34.90 (12.485)	2.60 (10.814)	37.57 (12.500)	3.52 (8.234)
Median	47.00	6.00	40.50	1.50	41.00	4.00
Min, Max	17.0, 52.0	-8.0, 13.0	8.0, 46.0	-14.0, 22.0	8.0, 52.0	-14.0, 22.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.1
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Female

Visit/Statistics	D->D (N = 34)		P->D (N = 17)		Total (N = 51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	8	8	6	6	14	14
Mean (SD)	45.13 (7.900)	6.75 (6.756)	35.17 (11.940)	1.33 (12.078)	40.86 (10.705)	4.43 (9.403)
Median	49.50	4.50	40.50	-2.50	42.50	3.50
Min, Max	32.0, 52.0	1.0, 22.0	14.0, 44.0	-9.0, 23.0	14.0, 52.0	-9.0, 23.0
Week 104						
n	3	3	2	2	5	5
Mean (SD)	39.33 (10.214)	4.00 (16.523)	39.00 (1.414)	8.00 (18.385)	39.20 (7.259)	5.60 (15.027)
Median	35.00	3.00	39.00	8.00	38.00	3.00
Min, Max	32.0, 51.0	-12.0, 21.0	38.0, 40.0	-5.0, 21.0	32.0, 51.0	-12.0, 21.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1.2
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	40		19		59	
Mean (SD)	33.25 (12.208)		31.11 (11.070)		32.56 (11.801)	
Median	34.50		31.00		34.00	
Min, Max	6.0, 52.0		12.0, 52.0		6.0, 52.0	
Week 2						
n	41	40	20	19		
Mean (SD)	39.39 (10.950)	6.25 (8.582)	34.00 (9.798)	2.89 (8.768)		
Median	43.00	7.00	34.50	2.00		
Min, Max	9.0, 52.0	-8.0, 33.0	15.0, 52.0	-11.0, 25.0		
Week 4						
n	41	40	19	19		
Mean (SD)	40.61 (10.435)	7.55 (10.168)	35.68 (11.056)	4.58 (9.112)		
Median	44.00	6.92	37.00	4.00		
Min, Max	9.0, 52.0	-14.0, 36.0	16.0, 52.0	-12.0, 25.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
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Table GERMANY.2.6.3.1.2
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and
Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	32	31	14	14		
Mean (SD)	42.49 (7.422)	9.73 (8.587)	35.93 (10.141)	3.93 (12.431)		
Median	42.00	8.83	36.00	5.50		
Min, Max	21.7, 52.0	-1.0, 36.0	21.0, 52.0	-20.0, 23.0		
Week 12						
n	39	38	19	19		
Mean (SD)	42.64 (8.270)	9.42 (7.791)	35.74 (11.145)	4.63 (9.370)		
Median	43.00	7.50	38.00	5.00		
Min, Max	20.0, 52.0	-3.0, 26.0	10.0, 52.0	-9.0, 26.0		
Week 14						
n	38	37	18	18		
Mean (SD)	43.55 (7.783)	10.05 (8.870)	37.32 (11.685)	5.15 (10.082)		
Median	44.00	7.83	41.50	5.88		
Min, Max	19.0, 52.0	0.0, 35.0	16.0, 52.0	-15.0, 21.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.6.3.1.2
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and
Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	39	38	18	18		
Mean (SD)	42.15 (9.472)	8.94 (10.551)	40.44 (10.153)	8.28 (8.057)		
Median	43.00	7.50	43.50	9.50		
Min, Max	4.0, 52.0	-13.2, 35.0	20.0, 52.0	-12.0, 20.0		
Week 20						
n	34	33	14	14		
Mean (SD)	40.44 (11.537)	6.06 (9.346)	39.00 (13.536)	5.86 (11.072)		
Median	43.00	5.00	42.00	6.50		
Min, Max	4.0, 52.0	-13.0, 36.0	9.0, 52.0	-24.0, 19.0		
Week 24						
n	37	36	18	18		
Mean (SD)	41.46 (10.145)	7.91 (9.435)	40.50 (12.113)	8.33 (9.299)		
Median	44.00	6.00	45.00	10.50		
Min, Max	7.0, 52.0	-10.0, 36.0	13.0, 52.0	-13.0, 19.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.6.3.1.2
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and
Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	32	32	16	16	48	48
Mean (SD)	41.84 (10.399)	7.81 (8.638)	36.00 (12.404)	2.25 (9.781)	39.90 (11.322)	5.96 (9.315)
Median	45.00	7.50	35.50	0.00	43.00	6.50
Min, Max	10.0, 52.0	-9.0, 27.0	9.0, 52.0	-15.0, 15.0	9.0, 52.0	-15.0, 27.0
Week 56						
n	25	25	15	15	40	40
Mean (SD)	39.52 (10.421)	7.04 (8.720)	41.00 (5.606)	6.73 (10.457)	40.08 (8.868)	6.93 (9.277)
Median	40.00	8.00	42.00	5.00	41.50	6.50
Min, Max	17.0, 52.0	-6.0, 31.0	27.0, 50.0	-10.0, 25.0	17.0, 52.0	-10.0, 31.0
Week 72						
n	15	15	10	10	25	25
Mean (SD)	41.87 (11.319)	8.20 (9.473)	39.00 (8.882)	4.60 (9.119)	40.72 (10.314)	6.76 (9.315)
Median	46.00	7.00	41.50	5.00	44.00	7.00
Min, Max	17.0, 52.0	-8.0, 31.0	21.0, 49.0	-8.0, 22.0	17.0, 52.0	-8.0, 31.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Table GERMANY.2.6.3.1.2
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and
Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

< 65 Years

Visit/Statistics	D->D (N = 41)		P->D (N = 20)		Total (N = 61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	8	8	6	6	14	14
Mean (SD)	46.25 (6.606)	9.00 (7.091)	41.50 (7.842)	4.17 (11.427)	44.21 (7.287)	6.93 (9.135)
Median	48.00	8.50	42.50	3.00	45.00	7.50
Min, Max	32.0, 52.0	0.0, 22.0	28.0, 52.0	-9.0, 23.0	28.0, 52.0	-9.0, 23.0
Week 104						
n	3	3	2	2	5	5
Mean (SD)	42.33 (9.609)	-3.67 (7.638)	39.00 (1.414)	8.00 (18.385)	41.00 (7.071)	1.00 (12.430)
Median	44.00	-2.00	39.00	8.00	40.00	-2.00
Min, Max	32.0, 51.0	-12.0, 3.0	38.0, 40.0	-5.0, 21.0	32.0, 51.0	-12.0, 21.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1.2
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean (SD)	35.95 (8.504)		38.33 (9.245)		36.60 (8.555)	
Median	38.00		42.00		38.50	
Min, Max	14.0, 49.0		22.0, 46.0		14.0, 49.0	
Week 2						
n	15	15	5	5		
Mean (SD)	39.26 (7.717)	3.04 (2.940)	35.00 (10.223)	-1.80 (5.263)		
Median	41.00	2.00	39.00	-1.00		
Min, Max	21.0, 49.0	-1.0, 8.0	17.0, 42.0	-8.0, 6.0		
Week 4						
n	16	16	6	6		
Mean (SD)	41.31 (6.770)	5.36 (6.479)	36.00 (8.124)	-2.33 (8.937)		
Median	43.00	5.42	37.50	-2.50		
Min, Max	31.0, 50.0	-4.0, 20.0	22.0, 46.0	-13.0, 13.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.6.3.1.2
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and
Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	13	13	5	5		
Mean (SD)	38.85 (8.905)	2.45 (6.095)	32.65 (9.447)	-4.15 (6.480)		
Median	41.00	3.00	35.00	-5.75		
Min, Max	17.0, 48.0	-8.0, 14.0	16.3, 39.0	-10.0, 6.0		
Week 12						
n	13	13	6	6		
Mean (SD)	40.64 (5.632)	4.85 (6.369)	34.33 (9.791)	-4.00 (6.132)		
Median	42.00	3.27	37.50	-7.00		
Min, Max	30.0, 47.3	-2.0, 22.0	15.0, 42.0	-10.0, 6.0		
Week 14						
n	14	14	6	6		
Mean (SD)	41.43 (6.123)	5.06 (5.981)	38.33 (6.218)	0.00 (5.367)		
Median	42.50	3.92	38.50	1.50		
Min, Max	29.0, 49.0	-3.0, 17.0	28.0, 45.0	-9.0, 6.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.6.3.1.2
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	14	14	5	5		
Mean (SD)	40.50 (6.035)	4.13 (5.080)	39.60 (8.264)	0.20 (5.975)		
Median	41.50	3.92	42.00	0.00		
Min, Max	27.0, 47.0	-3.0, 13.0	27.0, 47.0	-9.0, 6.0		
Week 20						
n	13	13	6	6		
Mean (SD)	41.54 (5.577)	3.45 (4.911)	36.33 (7.421)	-2.00 (12.837)		
Median	43.00	2.00	35.50	-1.50		
Min, Max	30.0, 48.0	-3.0, 12.0	25.0, 45.0	-21.0, 11.0		
Week 24						
n	13	13	6	6		
Mean (SD)	39.46 (8.657)	3.68 (5.558)	36.00 (7.127)	-2.33 (5.888)		
Median	41.00	3.83	36.00	-0.50		
Min, Max	21.0, 48.0	-7.0, 11.0	24.0, 45.0	-10.0, 4.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1.2
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and
Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	13	13	5	5	18	18
Mean (SD)	39.23 (8.418)	2.99 (6.160)	31.60 (13.353)	-5.40 (12.582)	37.11 (10.215)	0.66 (8.887)
Median	43.00	4.00	38.00	-5.00	39.00	3.50
Min, Max	25.0, 48.0	-8.0, 14.0	14.0, 45.0	-25.0, 7.0	14.0, 48.0	-25.0, 14.0
Week 56						
n	11	11	2	2	13	13
Mean (SD)	38.64 (9.298)	2.35 (5.645)	38.50 (2.121)	-6.00 (4.243)	38.62 (8.510)	1.06 (6.155)
Median	42.00	1.83	38.50	-6.00	40.00	0.00
Min, Max	23.0, 49.0	-4.0, 14.0	37.0, 40.0	-9.0, -3.0	23.0, 49.0	-9.0, 14.0
Week 72						
n	6	6	2	2	8	8
Mean (SD)	40.67 (8.214)	3.33 (5.203)	25.00 (24.042)	-9.00 (7.071)	36.75 (13.541)	0.25 (7.686)
Median	42.50	2.50	25.00	-9.00	41.00	1.00
Min, Max	27.0, 49.0	-2.0, 13.0	8.0, 42.0	-14.0, -4.0	8.0, 49.0	-14.0, 13.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.2
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and
Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

≥ 65 Years

Visit/Statistics	D->D (N = 16)		P->D (N = 6)		Total (N = 22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	2	2	8	8
Mean (SD)	44.83 (5.601)	7.50 (7.609)	24.00 (14.142)	-10.00 (2.828)	39.63 (11.999)	3.13 (10.398)
Median	47.00	6.00	24.00	-10.00	43.00	3.50
Min, Max	36.0, 50.0	1.0, 22.0	14.0, 34.0	-12.0, -8.0	14.0, 50.0	-12.0, 22.0
Week 104						
n	1	1	0	0	1	1
Mean (SD)	35.00 (NA)	21.00 (NA)			35.00 (NA)	21.00 (NA)
Median	35.00	21.00			35.00	21.00
Min, Max	35.0, 35.0	21.0, 21.0			35.0, 35.0	21.0, 21.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1.3
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and
Period 2 and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		13		37	
Mean (SD)	33.10 (10.080)		30.31 (8.077)		32.12 (9.407)	
Median	34.50		29.00		33.00	
Min, Max	14.0, 49.0		17.0, 45.0		14.0, 49.0	
Week 2						
n	24	23	13	13		
Mean (SD)	37.28 (12.045)	4.24 (7.335)	32.00 (8.888)	1.69 (9.114)		
Median	41.00	3.83	33.00	0.00		
Min, Max	9.0, 51.0	-7.0, 25.0	17.0, 49.0	-11.0, 25.0		
Week 4						
n	25	24	13	13		
Mean (SD)	38.40 (11.630)	5.53 (9.053)	34.77 (11.204)	4.46 (7.975)		
Median	41.00	6.42	35.00	3.00		
Min, Max	9.0, 51.0	-14.0, 27.0	16.0, 49.0	-6.0, 21.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.3
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and
Period 2 and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	19	18	8	8		
Mean (SD)	41.47 (8.422)	7.87 (7.873)	34.16 (10.980)	2.78 (13.023)		
Median	43.00	7.92	36.00	0.50		
Min, Max	17.0, 52.0	-8.0, 28.0	16.3, 47.0	-13.0, 23.0		
Week 12						
n	23	22	13	13		
Mean (SD)	41.48 (6.940)	8.71 (6.474)	31.31 (11.849)	1.00 (8.396)		
Median	42.00	7.50	32.00	0.00		
Min, Max	22.0, 52.0	-2.0, 22.0	10.0, 45.0	-9.0, 16.0		
Week 14						
n	23	22	13	13		
Mean (SD)	41.52 (8.877)	7.76 (6.506)	34.52 (11.435)	4.21 (10.302)		
Median	44.00	7.00	39.00	5.75		
Min, Max	19.0, 52.0	-3.0, 21.0	16.0, 48.0	-15.0, 21.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.3.1.3
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and
Period 2 and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	24	23	13	13		
Mean (SD)	39.13 (10.584)	5.81 (8.949)	38.77 (9.825)	8.46 (8.530)		
Median	41.00	7.00	42.00	10.00		
Min, Max	4.0, 52.0	-13.2, 22.0	21.0, 51.0	-12.0, 20.0		
Week 20						
n	20	19	9	9		
Mean (SD)	39.90 (11.121)	3.88 (6.768)	36.44 (13.584)	5.44 (13.220)		
Median	43.00	4.00	41.00	11.00		
Min, Max	4.0, 52.0	-13.0, 13.0	9.0, 49.0	-24.0, 19.0		
Week 24						
n	21	20	13	13		
Mean (SD)	37.90 (11.975)	4.48 (6.590)	38.62 (12.366)	8.31 (10.218)		
Median	43.00	3.42	44.00	12.00		
Min, Max	7.0, 52.0	-10.0, 17.0	13.0, 52.0	-13.0, 19.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.3
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	12	12	30	30
Mean (SD)	37.33 (11.576)	5.49 (6.475)	33.50 (12.296)	2.67 (11.023)	35.80 (11.813)	4.36 (8.523)
Median	39.00	4.42	34.00	4.00	36.00	4.42
Min, Max	10.0, 52.0	-9.0, 14.0	9.0, 52.0	-15.0, 15.0	9.0, 52.0	-15.0, 15.0
Week 56						
n	13	13	11	11	24	24
Mean (SD)	36.15 (12.727)	4.91 (6.390)	40.18 (5.456)	9.00 (10.227)	38.00 (10.082)	6.78 (8.433)
Median	42.00	3.00	42.00	11.00	42.00	4.50
Min, Max	17.0, 52.0	-4.0, 16.0	27.0, 48.0	-6.0, 25.0	17.0, 52.0	-6.0, 25.0
Week 72						
n	8	8	8	8	16	16
Mean (SD)	38.88 (12.076)	5.75 (7.285)	33.00 (13.384)	2.13 (11.850)	35.94 (12.683)	3.94 (9.685)
Median	43.00	8.00	36.00	0.50	40.00	5.50
Min, Max	17.0, 52.0	-8.0, 13.0	8.0, 46.0	-14.0, 22.0	8.0, 52.0	-14.0, 22.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.3.1.3
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Europe

Visit/Statistics	D->D (N = 25)		P->D (N = 13)		Total (N = 38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	5	5	11	11
Mean (SD)	46.50 (5.431)	11.00 (7.071)	34.00 (12.961)	2.40 (13.183)	40.82 (11.161)	7.09 (10.709)
Median	48.00	10.00	40.00	-1.00	44.00	8.00
Min, Max	36.0, 51.0	1.0, 22.0	14.0, 44.0	-9.0, 23.0	14.0, 51.0	-9.0, 23.0
Week 104						
n	1	1	2	2	3	3
Mean (SD)	35.00 (NA)	21.00 (NA)	39.00 (1.414)	8.00 (18.385)	37.67 (2.517)	12.33 (15.011)
Median	35.00	21.00	39.00	8.00	38.00	21.00
Min, Max	35.0, 35.0	21.0, 21.0	38.0, 40.0	-5.0, 21.0	35.0, 40.0	-5.0, 21.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1.3
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and
Period 2 and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	34.72 (12.190)		35.58 (13.194)		34.95 (12.321)	
Median	39.00		42.00		39.50	
Min, Max	6.0, 52.0		12.0, 52.0		6.0, 52.0	
Week 2						
n	32	32	12	11		
Mean (SD)	40.91 (8.255)	6.19 (7.769)	36.58 (10.300)	2.18 (7.640)		
Median	42.50	6.00	38.00	0.00		
Min, Max	15.0, 52.0	-8.0, 33.0	15.0, 52.0	-8.0, 19.0		
Week 4						
n	32	32	12	12		
Mean (SD)	42.69 (7.059)	7.97 (9.417)	36.83 (9.523)	1.25 (10.805)		
Median	45.00	6.00	37.50	-0.50		
Min, Max	25.0, 52.0	-3.0, 36.0	20.0, 52.0	-13.0, 25.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.3
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and
Period 2 and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	26	26	11	11		
Mean (SD)	41.41 (7.760)	7.37 (9.136)	35.73 (9.371)	1.09 (11.031)		
Median	41.00	6.00	35.00	4.00		
Min, Max	21.7, 52.0	-8.0, 36.0	21.0, 52.0	-20.0, 20.0		
Week 12						
n	29	29	12	12		
Mean (SD)	42.66 (8.326)	7.91 (8.548)	39.83 (7.297)	4.25 (10.428)		
Median	44.00	5.00	39.00	3.50		
Min, Max	20.0, 52.0	-3.0, 26.0	26.0, 52.0	-10.0, 26.0		
Week 14						
n	29	29	11	11		
Mean (SD)	44.14 (5.835)	9.38 (9.690)	41.18 (8.280)	3.45 (8.454)		
Median	44.00	7.00	42.00	3.00		
Min, Max	33.0, 52.0	0.0, 35.0	21.0, 52.0	-9.0, 21.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.3.1.3
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and
Period 2 and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	29	29	10	10		
Mean (SD)	43.86 (6.093)	9.10 (9.983)	42.20 (9.449)	4.00 (7.572)		
Median	43.00	6.00	45.00	2.50		
Min, Max	31.0, 52.0	-2.0, 35.0	20.0, 52.0	-9.0, 16.0		
Week 20						
n	27	27	11	11		
Mean (SD)	41.37 (9.612)	6.33 (9.315)	39.64 (10.773)	1.91 (11.031)		
Median	43.00	5.00	41.00	4.00		
Min, Max	8.0, 52.0	-7.0, 36.0	18.0, 52.0	-21.0, 18.0		
Week 24						
n	29	29	11	11		
Mean (SD)	43.14 (7.175)	8.38 (9.734)	40.27 (9.941)	2.55 (8.395)		
Median	44.00	5.00	41.00	2.00		
Min, Max	21.0, 52.0	-7.0, 36.0	17.0, 52.0	-10.0, 17.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.6.3.1.3
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	9	9	36	36
Mean (SD)	43.59 (7.757)	7.04 (9.292)	36.89 (13.100)	-2.56 (10.088)	41.92 (9.623)	4.64 (10.254)
Median	45.00	6.00	40.00	-2.00	45.00	4.50
Min, Max	25.0, 52.0	-8.0, 27.0	18.0, 52.0	-25.0, 9.0	18.0, 52.0	-25.0, 27.0
Week 56						
n	23	23	6	6	29	29
Mean (SD)	41.00 (7.799)	6.00 (9.075)	41.67 (5.465)	-1.67 (8.335)	41.14 (7.293)	4.41 (9.333)
Median	40.00	4.00	42.00	-2.50	40.00	2.00
Min, Max	24.0, 52.0	-6.0, 31.0	35.0, 50.0	-10.0, 13.0	24.0, 52.0	-10.0, 31.0
Week 72						
n	13	13	4	4	17	17
Mean (SD)	43.15 (9.245)	7.46 (9.606)	44.00 (3.559)	2.75 (6.397)	43.35 (8.162)	6.35 (9.007)
Median	45.00	6.00	43.00	2.50	45.00	6.00
Min, Max	17.0, 52.0	-2.0, 31.0	41.0, 49.0	-4.0, 10.0	17.0, 52.0	-4.0, 31.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
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Table GERMANY.2.6.3.1.3
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Rest of World

Visit/Statistics	D->D (N = 32)		P->D (N = 13)		Total (N = 45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	8	8	3	3	11	11
Mean (SD)	45.00 (6.698)	6.38 (6.823)	42.33 (9.074)	-2.33 (10.599)	44.27 (7.030)	4.00 (8.462)
Median	47.00	4.50	41.00	-4.00	46.00	4.00
Min, Max	32.0, 52.0	0.0, 22.0	34.0, 52.0	-12.0, 9.0	32.0, 52.0	-12.0, 22.0
Week 104						
n	3	3	0	0	3	3
Mean (SD)	42.33 (9.609)	-3.67 (7.638)			42.33 (9.609)	-3.67 (7.638)
Median	44.00	-2.00			44.00	-2.00
Min, Max	32.0, 51.0	-12.0, 3.0			32.0, 51.0	-12.0, 3.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1.4

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		10		33	
Mean (SD)	34.61 (10.487)		31.00 (9.775)		33.52 (10.263)	
Median	35.00		30.00		34.00	
Min, Max	14.0, 52.0		15.0, 46.0		14.0, 52.0	
Week 2						
n	24	23	9	9		
Mean (SD)	39.70 (10.295)	5.30 (7.286)	35.78 (7.138)	6.44 (11.103)		
Median	43.00	6.00	37.00	8.00		
Min, Max	9.0, 52.0	-8.0, 25.0	26.0, 49.0	-11.0, 25.0		
Week 4						
n	24	23	10	10		
Mean (SD)	41.38 (9.263)	7.13 (7.759)	36.00 (10.729)	5.00 (10.306)		
Median	43.50	7.00	39.00	6.50		
Min, Max	14.0, 52.0	-4.0, 27.0	16.0, 49.0	-13.0, 21.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
 Change from baseline is summarized only for patients who have data at baseline and the specified time point.
 FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
 Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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 Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1.4
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	19	18	7	7		
Mean (SD)	40.00 (9.031)	5.39 (7.492)	37.14 (9.599)	9.00 (10.263)		
Median	39.00	5.50	39.00	11.00		
Min, Max	17.0, 52.0	-8.0, 20.0	21.0, 47.0	-10.0, 23.0		
Week 12						
n	22	21	10	10		
Mean (SD)	42.15 (7.920)	7.30 (7.609)	37.30 (7.088)	6.30 (7.334)		
Median	42.00	7.00	39.00	7.00		
Min, Max	22.0, 52.0	-3.0, 23.0	26.0, 45.0	-7.0, 16.0		
Week 14						
n	22	21	10	10		
Mean (SD)	42.82 (7.920)	7.76 (6.737)	36.50 (10.352)	5.50 (10.845)		
Median	44.00	7.00	38.00	6.00		
Min, Max	23.0, 52.0	-3.0, 21.0	16.0, 47.0	-9.0, 21.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Table GERMANY.2.6.3.1.4
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	22	21	9	9		
Mean (SD)	42.64 (7.436)	7.86 (7.009)	40.89 (8.536)	10.11 (7.737)		
Median	43.00	8.00	44.00	14.00		
Min, Max	27.0, 52.0	-2.0, 22.0	20.0, 48.0	0.0, 20.0		
Week 20						
n	17	16	9	9		
Mean (SD)	43.12 (8.366)	4.88 (5.071)	36.00 (11.619)	4.22 (11.777)		
Median	44.00	4.50	41.00	3.00		
Min, Max	23.0, 52.0	-3.0, 13.0	18.0, 48.0	-21.0, 19.0		
Week 24						
n	21	20	10	10		
Mean (SD)	40.86 (10.942)	5.75 (6.544)	36.50 (12.322)	5.50 (9.902)		
Median	44.00	6.00	39.50	5.50		
Min, Max	19.0, 52.0	-7.0, 20.0	13.0, 48.0	-10.0, 19.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.4
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	8	8	26	26
Mean (SD)	40.33 (12.797)	4.06 (6.430)	29.25 (11.055)	-4.63 (13.147)	36.92 (13.145)	1.38 (9.654)
Median	45.00	3.50	31.50	-6.00	39.00	3.00
Min, Max	10.0, 52.0	-9.0, 13.0	9.0, 42.0	-25.0, 13.0	9.0, 52.0	-25.0, 13.0
Week 56						
n	14	14	7	7	21	21
Mean (SD)	39.14 (12.508)	4.36 (6.629)	40.14 (3.288)	6.86 (10.976)	39.48 (10.255)	5.19 (8.134)
Median	43.50	4.00	41.00	13.00	42.00	4.00
Min, Max	17.0, 52.0	-6.0, 16.0	35.0, 44.0	-9.0, 20.0	17.0, 52.0	-9.0, 20.0
Week 72						
n	9	9	6	6	15	15
Mean (SD)	41.56 (11.928)	5.89 (5.487)	40.00 (5.762)	4.83 (11.635)	40.93 (9.684)	5.47 (8.114)
Median	46.00	6.00	41.50	3.50	42.00	6.00
Min, Max	17.0, 52.0	-1.0, 13.0	29.0, 46.0	-8.0, 22.0	17.0, 52.0	-8.0, 22.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Table GERMANY.2.6.3.1.4

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 24)		P->D (N = 10)		Total (N = 34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	7	3	3	10	10
Mean (SD)	46.86 (5.305)	9.57 (7.091)	40.67 (5.774)	-2.00 (9.539)	45.00 (5.925)	6.10 (9.219)
Median	48.00	8.00	44.00	-1.00	46.00	7.50
Min, Max	36.0, 52.0	0.0, 22.0	34.0, 44.0	-12.0, 7.0	34.0, 52.0	-12.0, 22.0
Week 104						
n	3	3	1	1	4	4
Mean (SD)	37.00 (6.245)	2.33 (16.921)	40.00 (NA)	-5.00 (NA)	37.75 (5.315)	0.50 (14.295)
Median	35.00	-2.00	40.00	-5.00	37.50	-3.50
Min, Max	32.0, 44.0	-12.0, 21.0	40.0, 40.0	-5.0, -5.0	32.0, 44.0	-12.0, 21.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.4

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		15		48	
Mean (SD)	33.62 (11.920)		34.07 (11.817)		33.76 (11.764)	
Median	37.00		37.00		37.00	
Min, Max	6.0, 51.0		12.0, 52.0		6.0, 52.0	
Week 2						
n	32	32	16	15		
Mean (SD)	39.09 (10.139)	5.43 (7.905)	33.31 (10.965)	-0.80 (4.632)		
Median	42.00	4.00	36.00	0.00		
Min, Max	11.0, 50.0	-7.0, 33.0	15.0, 52.0	-8.0, 7.0		
Week 4						
n	33	33	15	15		
Mean (SD)	40.39 (9.782)	6.78 (10.292)	35.60 (10.329)	1.53 (8.798)		
Median	44.00	5.00	36.00	0.00		
Min, Max	9.0, 52.0	-14.0, 36.0	18.0, 52.0	-12.0, 25.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
 Change from baseline is summarized only for patients who have data at baseline and the specified time point.
 FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
 Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
 D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
 Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
 Source: ADaM.ADSL, ADaM.ADQS
 Run Date: 2024-04-26T15:48:58
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Table GERMANY.2.6.3.1.4

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	26	26	12	12		
Mean (SD)	42.49 (7.061)	9.09 (9.040)	33.85 (10.148)	-2.40 (10.493)		
Median	43.00	6.42	33.50	-3.00		
Min, Max	21.7, 52.0	-2.0, 36.0	16.3, 52.0	-20.0, 20.0		
Week 12						
n	30	30	15	15		
Mean (SD)	42.13 (7.660)	8.92 (7.755)	34.13 (12.569)	0.07 (9.968)		
Median	43.50	6.42	36.00	-2.00		
Min, Max	20.0, 52.0	-2.0, 26.0	10.0, 52.0	-10.0, 26.0		
Week 14						
n	30	30	14	14		
Mean (SD)	43.10 (7.087)	9.32 (9.492)	38.34 (10.866)	2.70 (8.261)		
Median	44.00	7.00	41.50	3.50		
Min, Max	19.0, 52.0	-2.0, 35.0	18.0, 52.0	-15.0, 21.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.4
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	31	31	14	14		
Mean (SD)	41.06 (9.518)	7.51 (11.117)	39.86 (10.524)	4.21 (8.002)		
Median	43.00	6.00	42.50	5.50		
Min, Max	4.0, 52.0	-13.2, 35.0	21.0, 52.0	-12.0, 16.0		
Week 20						
n	30	30	11	11		
Mean (SD)	39.40 (10.997)	5.56 (9.751)	40.00 (12.361)	2.91 (12.486)		
Median	41.50	3.50	41.00	4.00		
Min, Max	4.0, 52.0	-13.0, 36.0	9.0, 52.0	-24.0, 18.0		
Week 24						
n	29	29	14	14		
Mean (SD)	41.00 (8.960)	7.51 (10.013)	41.43 (10.120)	5.79 (9.893)		
Median	43.00	3.83	44.50	5.50		
Min, Max	7.0, 52.0	-10.0, 36.0	20.0, 52.0	-13.0, 19.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1.4

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	13	13	40	40
Mean (SD)	41.59 (7.505)	7.99 (9.015)	38.46 (12.313)	3.54 (7.912)	40.58 (9.295)	6.55 (8.827)
Median	44.00	6.00	39.00	0.00	43.50	5.00
Min, Max	28.0, 52.0	-8.0, 27.0	14.0, 52.0	-8.0, 15.0	14.0, 52.0	-8.0, 27.0
Week 56						
n	22	22	10	10	32	32
Mean (SD)	39.32 (8.289)	6.40 (9.010)	41.10 (6.557)	4.10 (10.959)	39.88 (7.728)	5.68 (9.541)
Median	40.00	3.00	42.00	1.50	40.50	2.50
Min, Max	20.0, 52.0	-5.0, 31.0	27.0, 50.0	-10.0, 25.0	20.0, 52.0	-10.0, 31.0
Week 72						
n	12	12	6	6	18	18
Mean (SD)	41.50 (9.530)	7.50 (10.613)	33.33 (16.219)	-0.17 (8.424)	38.78 (12.322)	4.94 (10.372)
Median	44.00	6.50	38.00	1.50	43.50	5.00
Min, Max	17.0, 52.0	-8.0, 31.0	8.0, 49.0	-14.0, 9.0	8.0, 52.0	-14.0, 31.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.6.3.1.4

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N = 33)		P->D (N = 16)		Total (N = 49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	7	5	5	12	12
Mean (SD)	44.43 (6.828)	7.14 (7.381)	35.00 (14.491)	2.20 (13.664)	40.50 (11.197)	5.08 (10.202)
Median	48.00	4.00	40.00	-4.00	41.50	3.50
Min, Max	32.0, 51.0	1.0, 22.0	14.0, 52.0	-9.0, 23.0	14.0, 52.0	-9.0, 23.0
Week 104						
n	1	1	1	1	2	2
Mean (SD)	51.00 (NA)	3.00 (NA)	38.00 (NA)	21.00 (NA)	44.50 (9.192)	12.00 (12.728)
Median	51.00	3.00	38.00	21.00	44.50	12.00
Min, Max	51.0, 51.0	3.0, 3.0	38.0, 38.0	21.0, 21.0	38.0, 51.0	3.0, 21.0

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.5

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		15		49	
Mean (SD)	33.56 (10.399)		32.47 (11.886)		33.22 (10.761)	
Median	34.50		31.00		34.00	
Min, Max	9.0, 51.0		12.0, 52.0		9.0, 52.0	
Week 2						
n	34	33	15	15		
Mean (SD)	39.56 (9.232)	6.09 (8.334)	34.27 (10.208)	1.80 (7.739)		
Median	42.00	5.00	35.00	0.00		
Min, Max	9.0, 51.0	-7.0, 33.0	15.0, 52.0	-11.0, 19.0		
Week 4						
n	35	34	15	15		
Mean (SD)	41.09 (8.357)	7.76 (9.082)	35.40 (10.336)	2.93 (8.713)		
Median	42.00	7.00	37.00	3.00		
Min, Max	14.0, 52.0	-3.0, 36.0	16.0, 52.0	-9.0, 25.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.5

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	29	28	12	12		
Mean (SD)	39.82 (8.422)	6.56 (6.983)	34.52 (11.028)	1.69 (11.277)		
Median	40.00	6.00	34.50	2.00		
Min, Max	17.0, 52.0	-8.0, 20.0	16.3, 52.0	-13.0, 20.0		
Week 12						
n	32	31	15	15		
Mean (SD)	41.73 (7.303)	8.30 (7.460)	35.87 (11.019)	3.40 (10.722)		
Median	42.00	7.00	37.00	0.00		
Min, Max	22.0, 52.0	-3.0, 26.0	15.0, 52.0	-10.0, 26.0		
Week 14						
n	31	30	14	14		
Mean (SD)	43.03 (6.529)	9.27 (8.308)	37.64 (11.318)	3.71 (7.670)		
Median	44.00	7.00	41.00	5.50		
Min, Max	23.0, 52.0	0.0, 35.0	16.0, 52.0	-9.0, 18.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.5

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	32	31	14	14		
Mean (SD)	42.16 (6.807)	8.77 (8.675)	41.00 (9.356)	7.07 (7.760)		
Median	42.50	8.00	42.00	7.00		
Min, Max	25.0, 52.0	-2.0, 32.0	20.0, 52.0	-9.0, 19.0		
Week 20						
n	29	28	13	13		
Mean (SD)	42.41 (7.224)	7.86 (8.793)	38.77 (10.576)	5.08 (8.893)		
Median	44.00	6.00	41.00	3.00		
Min, Max	23.0, 52.0	-1.0, 36.0	18.0, 52.0	-10.0, 19.0		
Week 24						
n	30	29	14	14		
Mean (SD)	41.23 (8.982)	7.41 (8.970)	39.86 (12.907)	5.93 (9.294)		
Median	43.50	5.00	45.00	5.50		
Min, Max	19.0, 52.0	-7.0, 36.0	13.0, 52.0	-9.0, 19.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
 Change from baseline is summarized only for patients who have data at baseline and the specified time point.
 FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
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 Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1.5

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	12	12	41	41
Mean (SD)	40.03 (10.561)	5.93 (9.188)	35.00 (14.245)	0.42 (10.740)	38.56 (11.801)	4.32 (9.863)
Median	44.00	4.00	37.00	-2.00	42.00	4.00
Min, Max	10.0, 52.0	-9.0, 27.0	9.0, 52.0	-15.0, 15.0	9.0, 52.0	-15.0, 27.0
Week 56						
n	22	22	10	10	32	32
Mean (SD)	39.82 (10.150)	5.23 (9.018)	42.50 (3.779)	6.10 (9.049)	40.66 (8.690)	5.50 (8.890)
Median	41.50	3.00	42.50	3.00	42.00	3.00
Min, Max	17.0, 52.0	-6.0, 31.0	35.0, 50.0	-3.0, 20.0	17.0, 52.0	-6.0, 31.0
Week 72						
n	12	12	7	7	19	19
Mean (SD)	41.42 (9.405)	6.42 (10.799)	36.43 (14.010)	1.71 (10.078)	39.58 (11.207)	4.68 (10.515)
Median	44.00	3.50	41.00	6.00	42.00	4.00
Min, Max	17.0, 52.0	-8.0, 31.0	8.0, 49.0	-14.0, 12.0	8.0, 52.0	-14.0, 31.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.5

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N = 35)		P->D (N = 15)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	8	8	5	5	13	13
Mean (SD)	46.63 (3.998)	7.00 (6.803)	36.40 (15.258)	-0.40 (8.295)	42.69 (10.664)	4.15 (7.998)
Median	47.00	6.00	44.00	-1.00	46.00	5.00
Min, Max	40.0, 51.0	0.0, 22.0	14.0, 52.0	-9.0, 9.0	14.0, 52.0	-9.0, 22.0
Week 104						
n	2	2	1	1	3	3
Mean (SD)	47.50 (4.950)	0.50 (3.536)	40.00 (NA)	-5.00 (NA)	45.00 (5.568)	-1.33 (4.041)
Median	47.50	0.50	40.00	-5.00	44.00	-2.00
Min, Max	44.0, 51.0	-2.0, 3.0	40.0, 40.0	-5.0, -5.0	40.0, 51.0	-5.0, 3.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.
 Change from baseline is summarized only for patients who have data at baseline and the specified time point.
 FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
 Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
 D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
 Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1.5

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	34.74 (12.710)		33.40 (9.935)		34.32 (11.768)	
Median	39.00		35.00		37.00	
Min, Max	6.0, 52.0		17.0, 46.0		6.0, 52.0	
Week 2						
n	22	22	10	9		
Mean (SD)	39.04 (11.575)	4.30 (6.325)	34.10 (9.351)	2.11 (9.636)		
Median	44.00	5.50	37.00	0.00		
Min, Max	11.0, 52.0	-8.0, 15.0	19.0, 49.0	-8.0, 25.0		
Week 4						
n	22	22	10	10		
Mean (SD)	40.36 (11.270)	5.62 (9.592)	36.30 (10.698)	2.90 (10.796)		
Median	44.50	4.00	37.00	1.50		
Min, Max	9.0, 52.0	-14.0, 21.0	18.0, 47.0	-13.0, 21.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
 Change from baseline is summarized only for patients who have data at baseline and the specified time point.
 FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
 Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
 D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
 Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1.5

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	16	16	7	7		
Mean (SD)	44.38 (6.228)	9.35 (10.791)	36.00 (8.021)	2.00 (13.026)		
Median	44.50	6.42	39.00	5.00		
Min, Max	29.0, 52.0	-2.0, 36.0	25.0, 47.0	-20.0, 23.0		
Week 12						
n	20	20	10	10		
Mean (SD)	42.80 (8.433)	8.18 (8.159)	34.70 (10.625)	1.30 (7.243)		
Median	45.00	6.42	39.00	3.00		
Min, Max	20.0, 52.0	-2.0, 24.0	10.0, 43.0	-9.0, 12.0		
Week 14						
n	21	21	10	10		
Mean (SD)	42.90 (8.648)	7.84 (8.728)	37.48 (9.740)	4.08 (11.671)		
Median	45.00	7.00	41.00	3.50		
Min, Max	19.0, 52.0	-3.0, 34.0	18.0, 48.0	-15.0, 21.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.3.1.5

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	21	21	9	9		
Mean (SD)	41.05 (11.088)	5.98 (10.809)	39.11 (10.434)	5.67 (9.407)		
Median	43.00	6.00	44.00	6.00		
Min, Max	4.0, 52.0	-13.2, 35.0	21.0, 47.0	-12.0, 20.0		
Week 20						
n	18	18	7	7		
Mean (SD)	38.06 (13.515)	1.37 (5.937)	37.14 (14.904)	0.57 (16.511)		
Median	42.00	2.00	44.00	4.00		
Min, Max	4.0, 52.0	-13.0, 10.0	9.0, 49.0	-24.0, 18.0		
Week 24						
n	20	20	10	10		
Mean (SD)	40.50 (10.995)	5.88 (8.512)	38.70 (8.603)	5.30 (10.698)		
Median	43.00	4.42	38.50	5.50		
Min, Max	7.0, 52.0	-10.0, 24.0	20.0, 50.0	-13.0, 17.0		

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 FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
 Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Table GERMANY.2.6.3.1.5

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	16	16	9	9	25	25
Mean (SD)	43.00 (8.375)	7.30 (6.326)	34.89 (10.386)	0.44 (11.304)	40.08 (9.776)	4.83 (8.882)
Median	45.50	8.00	35.00	0.00	43.00	7.00
Min, Max	26.0, 52.0	-3.0, 21.0	18.0, 48.0	-25.0, 14.0	18.0, 52.0	-25.0, 21.0
Week 56						
n	14	14	7	7	21	21
Mean (SD)	38.36 (9.982)	6.20 (6.770)	38.14 (6.440)	4.00 (13.416)	38.29 (8.787)	5.47 (9.215)
Median	40.00	8.50	39.00	2.00	39.00	8.00
Min, Max	20.0, 52.0	-5.0, 16.0	27.0, 48.0	-10.0, 25.0	20.0, 52.0	-10.0, 25.0
Week 72						
n	9	9	5	5	14	14
Mean (SD)	41.67 (12.062)	7.33 (5.074)	37.00 (10.440)	3.20 (11.077)	40.00 (11.334)	5.86 (7.604)
Median	46.00	8.00	42.00	-1.00	45.00	6.50
Min, Max	17.0, 52.0	-1.0, 13.0	21.0, 46.0	-5.0, 22.0	17.0, 52.0	-5.0, 22.0

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FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Table GERMANY.2.6.3.1.5

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N = 22)		P->D (N = 11)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	3	3	9	9
Mean (SD)	44.33 (8.238)	10.17 (7.627)	38.33 (3.786)	2.33 (18.339)	42.33 (7.416)	7.56 (11.652)
Median	48.00	9.50	40.00	-4.00	41.00	8.00
Min, Max	32.0, 52.0	1.0, 22.0	34.0, 41.0	-12.0, 23.0	32.0, 52.0	-12.0, 23.0
Week 104						
n	2	2	1	1	3	3
Mean (SD)	33.50 (2.121)	4.50 (23.335)	38.00 (NA)	21.00 (NA)	35.00 (3.000)	10.00 (19.053)
Median	33.50	4.50	38.00	21.00	35.00	21.00
Min, Max	32.0, 35.0	-12.0, 21.0	38.0, 38.0	21.0, 21.0	32.0, 38.0	-12.0, 21.0

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Table GERMANY.2.6.3.1.6

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		11		32	
Mean (SD)	34.91 (9.633)		33.00 (9.176)		34.26 (9.374)	
Median	35.00		33.00		34.50	
Min, Max	16.0, 49.0		22.0, 46.0		16.0, 49.0	
Week 2						
n	21	21	11	10		
Mean (SD)	38.37 (10.558)	3.46 (7.140)	34.55 (9.564)	2.90 (9.410)		
Median	41.00	1.00	34.00	1.00		
Min, Max	9.0, 51.0	-7.0, 25.0	17.0, 49.0	-7.0, 25.0		
Week 4						
n	21	21	11	11		
Mean (SD)	38.81 (10.157)	3.90 (8.863)	35.91 (10.858)	2.91 (9.311)		
Median	41.00	1.00	35.00	3.00		
Min, Max	14.0, 51.0	-14.0, 27.0	16.0, 49.0	-13.0, 21.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.3.1.6

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	14	14	6	6		
Mean (SD)	40.93 (7.163)	5.49 (5.400)	34.38 (12.476)	2.54 (13.002)		
Median	38.50	5.00	36.00	0.00		
Min, Max	29.0, 52.0	-2.0, 18.0	16.3, 47.0	-13.0, 23.0		
Week 12						
n	17	17	11	11		
Mean (SD)	40.59 (9.111)	6.11 (5.843)	33.00 (11.454)	0.00 (8.012)		
Median	42.00	4.83	32.00	-1.00		
Min, Max	22.0, 52.0	-2.0, 20.0	15.0, 51.0	-9.0, 13.0		
Week 14						
n	17	17	11	11		
Mean (SD)	41.35 (8.177)	5.87 (5.885)	34.73 (11.858)	1.73 (10.919)		
Median	42.00	6.00	37.00	5.00		
Min, Max	23.0, 52.0	-3.0, 21.0	16.0, 50.0	-15.0, 21.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
 Change from baseline is summarized only for patients who have data at baseline and the specified time point.
 FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
 Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
 D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
 Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1.6

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	18	18	11	11		
Mean (SD)	41.22 (7.635)	6.21 (6.960)	40.27 (9.572)	7.27 (8.900)		
Median	41.00	5.50	43.00	9.00		
Min, Max	25.0, 52.0	-3.0, 22.0	21.0, 52.0	-12.0, 20.0		
Week 20						
n	15	15	8	8		
Mean (SD)	40.60 (9.085)	3.59 (5.056)	33.50 (14.521)	0.38 (15.454)		
Median	43.00	2.83	37.00	4.50		
Min, Max	23.0, 52.0	-3.0, 13.0	9.0, 52.0	-24.0, 15.0		
Week 24						
n	15	15	11	11		
Mean (SD)	38.27 (11.774)	3.12 (6.056)	37.55 (13.148)	4.55 (11.049)		
Median	43.00	2.00	40.00	7.00		
Min, Max	19.0, 52.0	-7.0, 15.0	13.0, 52.0	-13.0, 19.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.3.1.6

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	10	10	27	27
Mean (SD)	37.35 (12.052)	3.75 (6.086)	32.10 (14.348)	-1.80 (13.307)	35.41 (12.936)	1.70 (9.569)
Median	38.00	4.00	34.00	-1.00	36.00	3.00
Min, Max	10.0, 52.0	-9.0, 14.0	9.0, 52.0	-25.0, 15.0	9.0, 52.0	-25.0, 15.0
Week 56						
n	13	13	10	10	23	23
Mean (SD)	37.15 (11.639)	3.60 (6.189)	39.80 (5.051)	5.70 (10.242)	38.30 (9.281)	4.51 (8.058)
Median	39.00	2.00	41.50	3.50	41.00	2.00
Min, Max	17.0, 52.0	-4.0, 14.0	27.0, 44.0	-9.0, 20.0	17.0, 52.0	-9.0, 20.0
Week 72						
n	7	7	8	8	15	15
Mean (SD)	40.86 (12.321)	3.71 (6.550)	38.13 (13.206)	2.88 (11.495)	39.40 (12.420)	3.27 (9.200)
Median	45.00	4.00	42.00	1.50	42.00	4.00
Min, Max	17.0, 52.0	-8.0, 11.0	8.0, 49.0	-14.0, 22.0	8.0, 52.0	-14.0, 22.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.
 Change from baseline is summarized only for patients who have data at baseline and the specified time point.
 FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
 Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
 D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
 Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1.6

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N = 21)		P->D (N = 12)		Total (N = 33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	5	5	5	5	10	10
Mean (SD)	46.40 (8.142)	4.80 (3.194)	34.40 (14.656)	-4.20 (8.408)	40.40 (12.843)	0.30 (7.646)
Median	50.00	4.00	34.00	-8.00	46.00	2.00
Min, Max	32.0, 51.0	1.0, 9.0	14.0, 52.0	-12.0, 9.0	14.0, 52.0	-12.0, 9.0
Week 104						
n	1	1	1	1	2	2
Mean (SD)	51.00 (NA)	3.00 (NA)	40.00 (NA)	-5.00 (NA)	45.50 (7.778)	-1.00 (5.657)
Median	51.00	3.00	40.00	-5.00	45.50	-1.00
Min, Max	51.0, 51.0	3.0, 3.0	40.0, 40.0	-5.0, -5.0	40.0, 51.0	-5.0, 3.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.
 Change from baseline is summarized only for patients who have data at baseline and the specified time point.
 FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
 Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
 D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
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Table GERMANY.2.6.3.1.6

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		14		49	
Mean (SD)	33.49 (12.244)		32.71 (12.493)		33.27 (12.190)	
Median	38.00		35.00		37.00	
Min, Max	6.0, 52.0		12.0, 52.0		6.0, 52.0	
Week 2						
n	35	34	14	14		
Mean (SD)	39.94 (9.953)	6.55 (7.710)	33.93 (10.111)	1.21 (7.688)		
Median	43.00	6.50	37.00	0.00		
Min, Max	11.0, 52.0	-8.0, 33.0	15.0, 52.0	-11.0, 19.0		
Week 4						
n	36	35	14	14		
Mean (SD)	41.97 (9.028)	8.74 (9.135)	35.64 (10.195)	2.93 (9.786)		
Median	45.00	7.83	37.50	0.50		
Min, Max	9.0, 52.0	-8.0, 36.0	18.0, 52.0	-12.0, 25.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
 Change from baseline is summarized only for patients who have data at baseline and the specified time point.
 FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
 Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
 D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
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Table GERMANY.2.6.3.1.6
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	31	30	13	13		
Mean (SD)	41.67 (8.386)	8.55 (9.603)	35.38 (8.903)	1.46 (11.428)		
Median	42.00	7.00	35.00	5.00		
Min, Max	17.0, 52.0	-8.0, 36.0	21.0, 52.0	-20.0, 20.0		
Week 12						
n	35	34	14	14		
Mean (SD)	42.89 (6.924)	9.33 (8.295)	37.29 (9.996)	4.57 (10.143)		
Median	43.00	7.00	38.50	3.00		
Min, Max	20.0, 52.0	-3.0, 26.0	10.0, 52.0	-10.0, 26.0		
Week 14						
n	35	34	13	13		
Mean (SD)	43.77 (6.941)	10.08 (9.204)	39.98 (8.891)	5.67 (7.674)		
Median	44.00	7.92	42.00	4.00		
Min, Max	19.0, 52.0	0.0, 35.0	21.0, 52.0	-4.0, 21.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.3.1.6

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	35	34	12	12		
Mean (SD)	41.97 (9.256)	8.41 (10.747)	40.25 (10.056)	5.83 (7.964)		
Median	43.00	7.00	43.50	5.00		
Min, Max	4.0, 52.0	-13.2, 35.0	20.0, 52.0	-9.0, 19.0		
Week 20						
n	32	31	12	12		
Mean (SD)	40.81 (10.808)	6.16 (9.528)	41.33 (9.129)	5.58 (8.918)		
Median	43.00	6.00	42.50	4.00		
Min, Max	4.0, 52.0	-13.0, 36.0	18.0, 52.0	-10.0, 19.0		
Week 24						
n	35	34	13	13		
Mean (SD)	42.09 (8.658)	8.41 (9.299)	40.92 (9.323)	6.62 (8.704)		
Median	44.00	7.00	45.00	4.00		
Min, Max	7.0, 52.0	-10.0, 36.0	17.0, 52.0	-9.0, 19.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	28	28	11	11	39	39
Mean (SD)	43.36 (7.602)	8.04 (9.020)	37.55 (10.425)	2.45 (7.764)	41.72 (8.757)	6.46 (8.953)
Median	45.00	7.00	40.00	0.00	44.00	6.00
Min, Max	26.0, 52.0	-8.0, 27.0	18.0, 52.0	-8.0, 14.0	18.0, 52.0	-8.0, 27.0
Week 56						
n	23	23	7	7	30	30
Mean (SD)	40.43 (8.948)	6.74 (8.971)	42.00 (5.859)	4.57 (12.150)	40.80 (8.265)	6.23 (9.616)
Median	41.00	4.00	42.00	-2.00	41.50	4.00
Min, Max	20.0, 52.0	-6.0, 31.0	35.0, 50.0	-10.0, 25.0	20.0, 52.0	-10.0, 31.0
Week 72						
n	14	14	4	4	18	18
Mean (SD)	41.86 (9.694)	8.36 (9.345)	33.75 (10.689)	1.25 (7.632)	40.06 (10.201)	6.78 (9.290)
Median	45.50	6.50	35.00	1.50	43.50	6.00
Min, Max	17.0, 52.0	-2.0, 31.0	21.0, 44.0	-8.0, 10.0	17.0, 52.0	-8.0, 31.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.6.3.1.6

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N = 36)		P->D (N = 14)		Total (N = 50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	9	9	3	3	12	12
Mean (SD)	45.22 (4.994)	10.33 (7.984)	41.67 (2.082)	8.67 (13.577)	44.33 (4.638)	9.92 (8.969)
Median	46.00	8.00	41.00	7.00	45.00	8.00
Min, Max	36.0, 52.0	0.0, 22.0	40.0, 44.0	-4.0, 23.0	36.0, 52.0	-4.0, 23.0
Week 104						
n	3	3	1	1	4	4
Mean (SD)	37.00 (6.245)	2.33 (16.921)	38.00 (NA)	21.00 (NA)	37.25 (5.123)	7.00 (16.673)
Median	35.00	-2.00	38.00	21.00	36.50	9.50
Min, Max	32.0, 44.0	-12.0, 21.0	38.0, 38.0	21.0, 21.0	32.0, 44.0	-12.0, 21.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.
 Change from baseline is summarized only for patients who have data at baseline and the specified time point.
 FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
 Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Table GERMANY.2.6.3.1.7

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	35.38 (8.847)		40.50 (5.260)		37.08 (7.982)	
Median	37.00		42.00		39.50	
Min, Max	20.0, 48.0		33.0, 45.0		20.0, 48.0	
Week 2						
n	8	8	4	4		
Mean (SD)	41.00 (7.111)	5.63 (5.502)	39.50 (2.082)	-1.00 (5.715)		
Median	40.00	6.50	39.50	-1.00		
Min, Max	33.0, 51.0	-1.0, 13.0	37.0, 42.0	-8.0, 6.0		
Week 4						
n	8	8	4	4		
Mean (SD)	41.75 (6.798)	6.38 (7.927)	40.25 (4.193)	-0.25 (9.359)		
Median	41.50	6.50	39.50	-2.50		
Min, Max	32.0, 50.0	-3.0, 22.0	36.0, 46.0	-9.0, 13.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
 Change from baseline is summarized only for patients who have data at baseline and the specified time point.
 FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
 Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Table GERMANY.2.6.3.1.7

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	8	8	4	4		
Mean (SD)	40.63 (7.745)	5.25 (8.447)	36.75 (2.630)	-3.75 (7.411)		
Median	40.50	6.50	37.00	-5.50		
Min, Max	27.0, 50.0	-8.0, 20.0	34.0, 39.0	-10.0, 6.0		
Week 12						
n	8	8	4	4		
Mean (SD)	41.88 (7.220)	6.50 (8.194)	38.00 (3.162)	-2.50 (7.326)		
Median	42.00	3.50	37.50	-3.00		
Min, Max	30.0, 51.0	-1.0, 23.0	35.0, 42.0	-10.0, 6.0		
Week 14						
n	8	8	4	4		
Mean (SD)	42.75 (5.548)	7.38 (6.391)	41.25 (4.113)	0.75 (2.872)		
Median	42.00	6.50	42.00	1.50		
Min, Max	35.0, 51.0	0.0, 19.0	36.0, 45.0	-3.0, 3.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	8	8	3	3		
Mean (SD)	42.75 (6.735)	7.38 (7.873)	41.67 (5.508)	-1.33 (7.506)		
Median	42.50	6.50	42.00	-1.00		
Min, Max	34.0, 52.0	-1.0, 23.0	36.0, 47.0	-9.0, 6.0		
Week 20						
n	8	8	4	4		
Mean (SD)	42.75 (6.985)	7.38 (7.405)	40.00 (5.228)	-0.50 (9.747)		
Median	42.50	6.00	40.00	-1.50		
Min, Max	30.0, 51.0	0.0, 23.0	35.0, 45.0	-10.0, 11.0		
Week 24						
n	8	8	4	4		
Mean (SD)	41.50 (9.769)	6.13 (7.338)	39.00 (4.967)	-1.50 (5.568)		
Median	43.00	6.00	38.50	-0.50		
Min, Max	21.0, 51.0	-7.0, 16.0	34.0, 45.0	-9.0, 4.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.7

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N = 8)		P->D (N = 4)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	42.67 (10.073)	6.50 (11.023)	41.00 (3.606)	2.00 (6.245)	42.11 (8.207)	5.00 (9.526)
Median	46.00	5.00	40.00	4.00	45.00	4.00
Min, Max	25.0, 51.0	-5.0, 25.0	38.0, 45.0	-5.0, 7.0	25.0, 51.0	-5.0, 25.0
Week 56						
n	4	4	1	1	5	5
Mean (SD)	40.50 (13.077)	1.25 (8.139)	40.00 (NA)	-3.00 (NA)	40.40 (11.327)	0.40 (7.301)
Median	43.50	-0.50	40.00	-3.00	40.00	-3.00
Min, Max	24.0, 51.0	-6.0, 12.0	40.0, 40.0	-3.0, -3.0	24.0, 51.0	-6.0, 12.0
Week 72						
n	1	1	0	0	1	1
Mean (SD)	48.00 (NA)	6.00 (NA)			48.00 (NA)	6.00 (NA)
Median	48.00	6.00			48.00	6.00
Min, Max	48.0, 48.0	6.0, 6.0			48.0, 48.0	6.0, 6.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.7

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	48		21		69	
Mean (SD)	33.80 (11.683)		31.38 (11.200)		33.06 (11.510)	
Median	36.00		31.00		35.00	
Min, Max	6.0, 52.0		12.0, 52.0		6.0, 52.0	
Week 2						
n	48	47	21	20		
Mean (SD)	39.08 (10.569)	5.33 (7.930)	33.19 (10.245)	2.50 (8.715)		
Median	42.50	4.00	34.00	1.00		
Min, Max	9.0, 52.0	-8.0, 33.0	15.0, 52.0	-11.0, 25.0		
Week 4						
n	49	48	21	21		
Mean (SD)	40.65 (9.913)	7.01 (9.535)	34.90 (10.913)	3.52 (9.490)		
Median	44.00	5.92	35.00	3.00		
Min, Max	9.0, 52.0	-14.0, 36.0	16.0, 52.0	-13.0, 25.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.
 Change from baseline is summarized only for patients who have data at baseline and the specified time point.
 FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
 Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
 D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
 Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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Table GERMANY.2.6.3.1.7

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	37	36	15	15		
Mean (SD)	41.61 (8.090)	8.09 (8.603)	34.62 (11.014)	3.28 (12.237)		
Median	42.00	6.00	33.00	5.00		
Min, Max	17.0, 52.0	-8.0, 36.0	16.3, 52.0	-20.0, 23.0		
Week 12						
n	44	43	21	21		
Mean (SD)	42.19 (7.855)	8.58 (7.615)	34.90 (11.536)	3.52 (9.553)		
Median	42.50	7.00	38.00	4.00		
Min, Max	20.0, 52.0	-3.0, 26.0	10.0, 52.0	-9.0, 26.0		
Week 14						
n	44	43	20	20		
Mean (SD)	43.02 (7.714)	8.92 (8.795)	36.84 (11.247)	4.49 (10.053)		
Median	44.00	7.00	41.00	5.88		
Min, Max	19.0, 52.0	-3.0, 35.0	16.0, 52.0	-15.0, 21.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.3.1.7

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	45	44	20	20		
Mean (SD)	41.53 (9.024)	7.70 (9.950)	40.05 (10.159)	7.70 (7.868)		
Median	43.00	6.50	43.50	7.50		
Min, Max	4.0, 52.0	-13.2, 35.0	20.0, 52.0	-12.0, 20.0		
Week 20						
n	39	38	16	16		
Mean (SD)	40.33 (10.756)	4.89 (8.579)	37.75 (13.138)	4.50 (12.415)		
Median	43.00	3.50	41.00	6.50		
Min, Max	4.0, 52.0	-13.0, 36.0	9.0, 52.0	-24.0, 19.0		
Week 24						
n	42	41	20	20		
Mean (SD)	40.83 (9.842)	6.92 (9.050)	39.45 (12.063)	7.10 (9.776)		
Median	43.50	3.83	44.50	8.50		
Min, Max	7.0, 52.0	-10.0, 36.0	13.0, 52.0	-13.0, 19.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.3.1.7

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	39	39	18	18	57	57
Mean (SD)	40.85 (9.922)	6.41 (7.907)	33.94 (13.153)	0.17 (11.388)	38.67 (11.393)	4.44 (9.506)
Median	44.00	5.00	34.00	-1.00	42.00	4.83
Min, Max	10.0, 52.0	-9.0, 27.0	9.0, 52.0	-25.0, 15.0	9.0, 52.0	-25.0, 27.0
Week 56						
n	32	32	16	16	48	48
Mean (SD)	39.09 (9.766)	6.15 (8.087)	40.75 (5.508)	5.75 (10.841)	39.65 (8.556)	6.02 (8.983)
Median	40.50	4.00	42.00	3.50	41.50	4.00
Min, Max	17.0, 52.0	-5.0, 31.0	27.0, 50.0	-10.0, 25.0	17.0, 52.0	-10.0, 31.0
Week 72						
n	20	20	12	12	32	32
Mean (SD)	41.20 (10.496)	6.85 (8.863)	36.67 (12.116)	2.33 (10.030)	39.50 (11.162)	5.16 (9.422)
Median	45.00	6.50	41.50	1.50	42.50	5.00
Min, Max	17.0, 52.0	-8.0, 31.0	8.0, 49.0	-14.0, 22.0	8.0, 52.0	-14.0, 31.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.7

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Patients Enrolled from Japan No

Visit/Statistics	D->D (N = 49)		P->D (N = 22)		Total (N = 71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	45.64 (6.008)	8.36 (7.067)	37.13 (11.753)	0.63 (11.722)	42.55 (9.272)	5.55 (9.551)
Median	48.00	7.50	40.50	-2.50	45.00	6.00
Min, Max	32.0, 52.0	0.0, 22.0	14.0, 52.0	-12.0, 23.0	14.0, 52.0	-12.0, 23.0
Week 104						
n	4	4	2	2	6	6
Mean (SD)	40.50 (8.660)	2.50 (13.820)	39.00 (1.414)	8.00 (18.385)	40.00 (6.782)	4.33 (13.794)
Median	39.50	0.50	39.00	8.00	39.00	0.50
Min, Max	32.0, 51.0	-12.0, 21.0	38.0, 40.0	-5.0, 21.0	32.0, 51.0	-12.0, 21.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.8

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	38.45 (9.521)		36.00 (13.300)		37.69 (10.681)	
Median	40.50		42.00		41.00	
Min, Max	20.0, 52.0		12.0, 52.0		12.0, 52.0	
Week 2						
n	22	22	10	10		
Mean (SD)	42.36 (6.107)	3.91 (6.625)	38.40 (9.913)	2.40 (8.017)		
Median	43.50	1.50	39.00	1.50		
Min, Max	28.0, 51.0	-8.0, 22.0	15.0, 52.0	-8.0, 19.0		
Week 4						
n	22	22	10	10		
Mean (SD)	43.59 (6.254)	5.14 (8.061)	38.90 (8.543)	2.90 (10.796)		
Median	45.00	3.00	38.50	2.00		
Min, Max	28.0, 52.0	-3.0, 25.0	21.0, 52.0	-12.0, 25.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.6.3.1.8
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	18	18	10	10		
Mean (SD)	42.67 (7.468)	4.44 (6.758)	36.60 (9.395)	0.60 (11.501)		
Median	41.50	2.50	37.00	2.00		
Min, Max	27.0, 52.0	-8.0, 20.0	21.0, 52.0	-20.0, 20.0		
Week 12						
n	20	20	10	10		
Mean (SD)	44.86 (5.937)	6.01 (8.689)	40.60 (7.691)	4.60 (10.585)		
Median	45.50	2.50	40.50	3.50		
Min, Max	30.0, 52.0	-3.0, 26.0	26.0, 52.0	-10.0, 26.0		
Week 14						
n	20	20	9	9		
Mean (SD)	44.95 (5.226)	6.10 (7.018)	41.56 (9.126)	2.89 (5.578)		
Median	45.00	3.00	44.00	3.00		
Min, Max	35.0, 52.0	0.0, 24.0	21.0, 52.0	-4.0, 14.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.6.3.1.8

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	20	20	8	8		
Mean (SD)	44.75 (5.618)	5.90 (7.913)	42.38 (10.433)	3.00 (7.051)		
Median	44.00	2.50	45.00	2.50		
Min, Max	34.0, 52.0	-2.0, 24.0	20.0, 52.0	-9.0, 14.0		
Week 20						
n	20	20	9	9		
Mean (SD)	43.90 (6.545)	5.05 (7.265)	41.33 (10.724)	2.67 (7.314)		
Median	44.00	2.00	44.00	4.00		
Min, Max	30.0, 52.0	-1.0, 24.0	18.0, 52.0	-10.0, 11.0		
Week 24						
n	20	20	9	9		
Mean (SD)	44.15 (7.257)	5.30 (7.512)	41.00 (10.954)	2.33 (6.500)		
Median	44.00	2.50	45.00	2.00		
Min, Max	21.0, 52.0	-7.0, 24.0	17.0, 52.0	-9.0, 14.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:49:01

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Table GERMANY.2.6.3.1.8

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	7	7	27	27
Mean (SD)	44.15 (7.548)	5.15 (9.207)	41.86 (9.924)	0.71 (6.264)	43.56 (8.088)	4.00 (8.656)
Median	45.50	3.00	43.00	0.00	45.00	3.00
Min, Max	25.0, 52.0	-8.0, 25.0	23.0, 52.0	-8.0, 9.0	23.0, 52.0	-8.0, 25.0
Week 56						
n	16	16	5	5	21	21
Mean (SD)	42.38 (7.429)	3.38 (7.848)	42.60 (5.550)	-0.20 (8.408)	42.43 (6.896)	2.52 (7.922)
Median	41.50	1.50	44.00	-2.00	42.00	1.00
Min, Max	24.0, 52.0	-6.0, 21.0	35.0, 50.0	-10.0, 13.0	24.0, 52.0	-10.0, 21.0
Week 72						
n	9	9	3	3	12	12
Mean (SD)	45.00 (5.099)	5.89 (7.557)	44.67 (4.041)	5.00 (5.568)	44.92 (4.680)	5.67 (6.880)
Median	45.00	4.00	44.00	6.00	45.00	5.00
Min, Max	36.0, 52.0	-2.0, 23.0	41.0, 49.0	-1.0, 10.0	36.0, 52.0	-2.0, 23.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.6.3.1.8
Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Asian

Visit/Statistics	D->D (N = 22)		P->D (N = 10)		Total (N = 32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	7	2	2	9	9
Mean (SD)	44.00 (6.557)	6.14 (7.335)	46.50 (7.778)	2.50 (9.192)	44.56 (6.405)	5.33 (7.314)
Median	46.00	4.00	46.50	2.50	46.00	4.00
Min, Max	32.0, 51.0	0.0, 22.0	41.0, 52.0	-4.0, 9.0	32.0, 52.0	-4.0, 22.0
Week 104						
n	2	2	0	0	2	2
Mean (SD)	47.50 (4.950)	0.50 (3.536)			47.50 (4.950)	0.50 (3.536)
Median	47.50	0.50			47.50	0.50
Min, Max	44.0, 51.0	-2.0, 3.0			44.0, 51.0	-2.0, 3.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.
FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Table GERMANY.2.6.3.1.8

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	27		10		37	
Mean (SD)	32.23 (10.456)		34.30 (8.125)		32.79 (9.815)	
Median	34.00		37.00		34.00	
Min, Max	6.0, 49.0		21.0, 46.0		6.0, 49.0	
Week 2						
n	27	26	10	9		
Mean (SD)	38.33 (11.032)	6.21 (6.511)	33.00 (6.146)	-0.11 (5.776)		
Median	43.00	7.00	34.50	0.00		
Min, Max	9.0, 52.0	-7.0, 25.0	21.0, 40.0	-11.0, 8.0		
Week 4						
n	28	27	10	10		
Mean (SD)	39.79 (10.020)	7.80 (8.617)	36.90 (9.315)	2.60 (8.449)		
Median	43.00	8.00	36.50	3.50		
Min, Max	14.0, 52.0	-14.0, 27.0	20.0, 49.0	-13.0, 12.0		

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FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Modified Randomized Set

White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	22	21	7	7		
Mean (SD)	40.91 (7.831)	8.98 (9.019)	33.86 (8.255)	1.57 (10.845)		
Median	40.50	8.83	33.00	5.00		
Min, Max	17.0, 52.0	-8.0, 36.0	24.0, 45.0	-13.0, 16.0		
Week 12						
n	25	24	10	10		
Mean (SD)	40.64 (8.597)	8.82 (5.976)	37.20 (8.149)	2.90 (8.875)		
Median	42.00	8.00	40.00	2.50		
Min, Max	20.0, 52.0	-2.0, 23.0	19.0, 45.0	-8.0, 16.0		
Week 14						
n	25	24	10	10		
Mean (SD)	42.68 (7.116)	10.03 (8.334)	39.80 (7.036)	5.50 (10.288)		
Median	43.00	8.00	41.50	5.50		
Min, Max	23.0, 52.0	-3.0, 34.0	23.0, 48.0	-9.0, 21.0		

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FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

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Modified Randomized Set

White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	26	25	10	10		
Mean (SD)	41.27 (7.097)	9.03 (9.708)	43.70 (4.715)	9.40 (6.552)		
Median	41.00	8.00	44.00	10.00		
Min, Max	25.0, 52.0	-13.2, 35.0	36.0, 51.0	0.0, 19.0		
Week 20						
n	21	20	8	8		
Mean (SD)	39.24 (10.521)	5.28 (5.129)	41.25 (7.498)	7.63 (13.437)		
Median	43.00	5.00	42.00	13.50		
Min, Max	8.0, 52.0	-3.2, 13.0	25.0, 49.0	-21.0, 19.0		
Week 24						
n	23	22	10	10		
Mean (SD)	39.70 (9.373)	7.48 (7.302)	44.60 (5.232)	10.30 (8.883)		
Median	40.00	7.50	45.00	12.50		
Min, Max	19.0, 52.0	-3.0, 24.0	36.0, 52.0	-10.0, 19.0		

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Modified Randomized Set

White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	10	10	28	28
Mean (SD)	39.11 (11.641)	6.27 (6.526)	35.40 (10.731)	1.10 (13.395)	37.79 (11.269)	4.42 (9.643)
Median	43.00	4.92	35.50	3.00	38.50	4.92
Min, Max	10.0, 52.0	-9.0, 19.0	18.0, 52.0	-25.0, 15.0	10.0, 52.0	-25.0, 19.0
Week 56						
n	15	15	8	8	23	23
Mean (SD)	38.67 (11.210)	6.32 (6.573)	41.13 (4.121)	4.50 (9.024)	39.52 (9.317)	5.69 (7.361)
Median	42.00	8.00	41.50	3.50	42.00	5.00
Min, Max	17.0, 52.0	-4.0, 16.0	35.0, 48.0	-9.0, 18.0	17.0, 52.0	-9.0, 18.0
Week 72						
n	9	9	6	6	15	15
Mean (SD)	39.22 (13.827)	4.44 (6.654)	38.50 (6.565)	0.17 (8.232)	38.93 (11.171)	2.73 (7.363)
Median	46.00	7.00	41.00	-3.50	42.00	2.00
Min, Max	17.0, 52.0	-8.0, 13.0	29.0, 46.0	-8.0, 12.0	17.0, 52.0	-8.0, 13.0

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Modified Randomized Set

White

Visit/Statistics	D->D (N = 28)		P->D (N = 11)		Total (N = 39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	4	4	10	10
Mean (SD)	49.17 (2.229)	8.67 (4.590)	37.50 (7.895)	-3.75 (8.539)	44.50 (7.735)	3.70 (8.782)
Median	49.00	8.50	39.00	-5.00	47.00	7.50
Min, Max	46.0, 52.0	1.0, 15.0	28.0, 44.0	-12.0, 7.0	28.0, 52.0	-12.0, 15.0
Week 104						
n	1	1	1	1	2	2
Mean (SD)	32.00 (NA)	-12.00 (NA)	40.00 (NA)	-5.00 (NA)	36.00 (5.657)	-8.50 (4.950)
Median	32.00	-12.00	40.00	-5.00	36.00	-8.50
Min, Max	32.0, 32.0	-12.0, -12.0	40.0, 40.0	-5.0, -5.0	32.0, 40.0	-12.0, -5.0

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Table GERMANY.2.6.3.1.8

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		5		12	
Mean (SD)	27.00 (15.111)		23.60 (5.857)		25.58 (11.836)	
Median	21.00		22.00		22.00	
Min, Max	9.0, 44.0		17.0, 33.0		9.0, 44.0	
Week 2						
n	7	7	5	5		
Mean (SD)	33.86 (14.392)	6.86 (13.184)	28.20 (12.755)	4.60 (12.857)		
Median	36.00	7.00	26.00	2.00		
Min, Max	11.0, 50.0	-7.0, 33.0	17.0, 49.0	-7.0, 25.0		
Week 4						
n	7	7	5	5		
Mean (SD)	36.14 (13.934)	9.14 (14.690)	27.20 (12.398)	3.60 (10.213)		
Median	41.00	6.00	22.00	1.00		
Min, Max	9.0, 50.0	-8.0, 36.0	16.0, 45.0	-6.0, 21.0		

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Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	5	5	2	2		
Mean (SD)	39.33 (11.078)	12.93 (9.722)	31.63 (21.744)	8.63 (20.329)		
Median	44.00	12.67	31.63	8.63		
Min, Max	21.7, 50.0	3.0, 28.0	16.3, 47.0	-5.8, 23.0		
Week 12						
n	7	7	5	5		
Mean (SD)	39.71 (7.410)	12.71 (8.480)	21.40 (8.820)	-2.20 (7.662)		
Median	37.00	16.00	24.00	-7.00		
Min, Max	28.0, 50.0	1.0, 22.0	10.0, 32.0	-9.0, 8.0		
Week 14						
n	7	7	5	5		
Mean (SD)	38.43 (11.660)	11.43 (11.458)	25.95 (11.614)	2.35 (13.633)		
Median	44.00	8.00	22.75	5.75		
Min, Max	19.0, 50.0	2.0, 35.0	16.0, 45.0	-15.0, 21.0		

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Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	7	7	5	5		
Mean (SD)	34.71 (15.913)	7.71 (13.732)	30.00 (9.823)	6.40 (12.095)		
Median	41.00	7.00	27.00	5.00		
Min, Max	4.0, 50.0	-13.0, 32.0	21.0, 44.0	-12.0, 20.0		
Week 20						
n	6	6	3	3		
Mean (SD)	35.50 (16.355)	6.33 (18.096)	20.67 (12.014)	-5.00 (17.692)		
Median	40.00	2.00	20.00	-2.00		
Min, Max	4.0, 50.0	-13.0, 36.0	9.0, 33.0	-24.0, 11.0		
Week 24						
n	7	7	5	5		
Mean (SD)	35.86 (14.645)	8.86 (15.170)	26.00 (10.512)	2.40 (13.594)		
Median	43.00	3.00	24.00	2.00		
Min, Max	7.0, 47.0	-10.0, 36.0	13.0, 39.0	-13.0, 17.0		

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:49:01

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Table GERMANY.2.6.3.1.8

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	7	7	4	4	11	11
Mean (SD)	37.43 (9.378)	10.43 (9.163)	21.75 (12.038)	-1.75 (11.786)	31.73 (12.602)	6.00 (11.393)
Median	36.00	9.00	22.50	-4.00	31.00	9.00
Min, Max	26.0, 50.0	-3.0, 27.0	9.0, 33.0	-13.0, 14.0	9.0, 50.0	-13.0, 27.0
Week 56						
n	5	5	4	4	9	9
Mean (SD)	31.00 (9.849)	10.60 (11.950)	37.50 (7.141)	13.50 (13.626)	33.89 (8.908)	11.89 (11.973)
Median	30.00	9.00	40.50	17.50	39.00	9.00
Min, Max	20.0, 42.0	1.0, 31.0	27.0, 42.0	-6.0, 25.0	20.0, 42.0	-6.0, 31.0
Week 72						
n	3	3	3	3	6	6
Mean (SD)	38.00 (10.149)	16.67 (12.897)	25.00 (19.313)	4.00 (18.000)	31.50 (15.527)	10.33 (15.629)
Median	40.00	13.00	21.00	4.00	33.50	9.50
Min, Max	27.0, 47.0	6.0, 31.0	8.0, 46.0	-14.0, 22.0	8.0, 47.0	-14.0, 31.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:49:01

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Table GERMANY.2.6.3.1.8

Summary of Actual Values and Change from Baseline in FACIT Fatigue Scores During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Others or Unknown

Visit/Statistics	D->D (N = 7)		P->D (N = 5)		Total (N = 12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	1	1	2	2	3	3
Mean (SD)	36.00 (NA)	22.00 (NA)	27.00 (18.385)	7.50 (21.920)	30.00 (14.000)	12.33 (17.616)
Median	36.00	22.00	27.00	7.50	36.00	22.00
Min, Max	36.0, 36.0	22.0, 22.0	14.0, 40.0	-8.0, 23.0	14.0, 40.0	-8.0, 23.0
Week 104						
n	1	1	1	1	2	2
Mean (SD)	35.00 (NA)	21.00 (NA)	38.00 (NA)	21.00 (NA)	36.50 (2.121)	21.00 (0.000)
Median	35.00	21.00	38.00	21.00	36.50	21.00
Min, Max	35.0, 35.0	21.0, 21.0	38.0, 38.0	21.0, 21.0	35.0, 38.0	21.0, 21.0

Baseline is defined as the last non-missing value prior to the first dose of study drug.
 Change from baseline is summarized only for patients who have data at baseline and the specified time point.
 FACIT-Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
 Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
 D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
 Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
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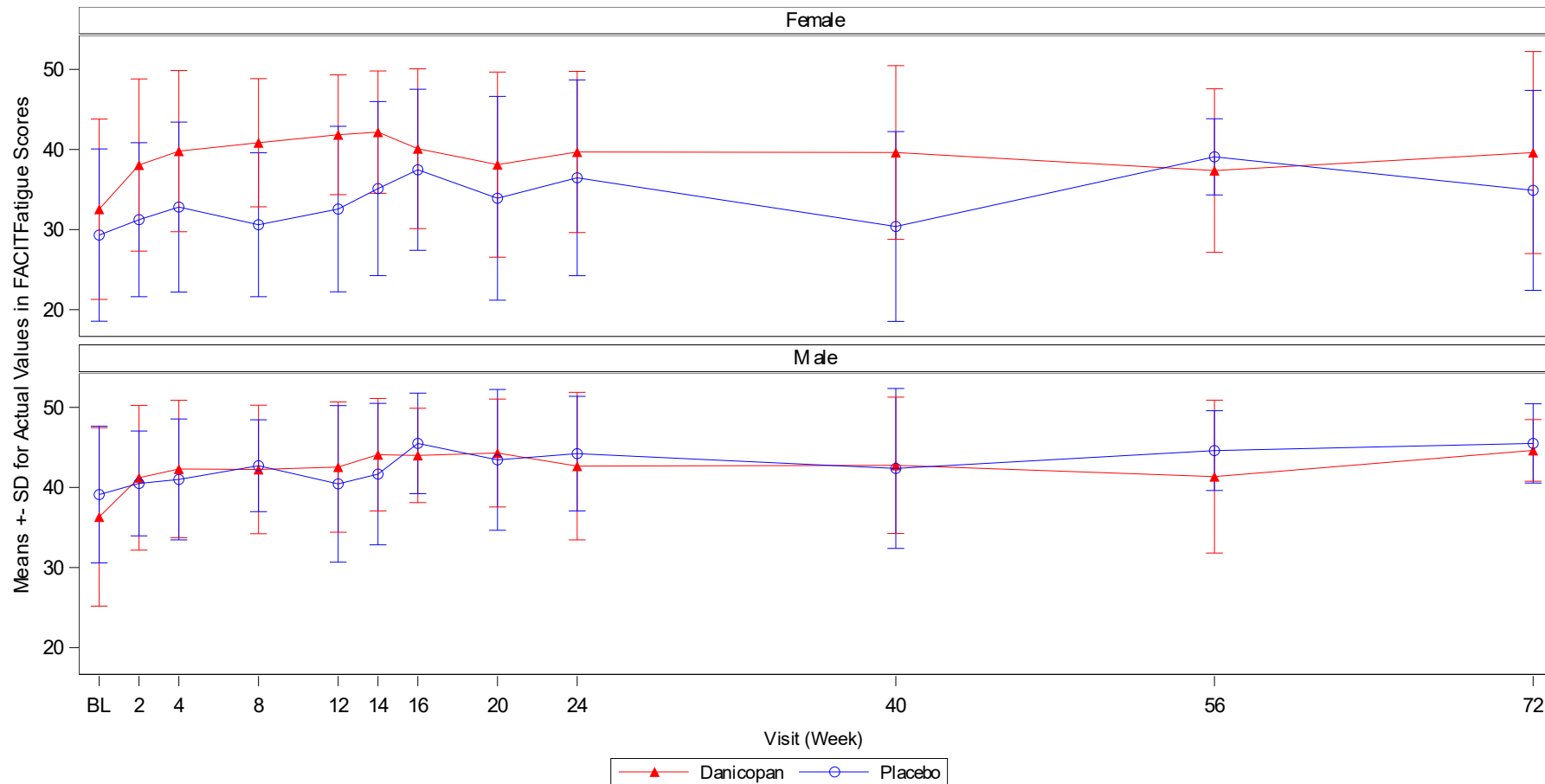
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Figure GERMANY.2.2.1.3.1
Plots of Mean \pm SD for Actual Values in FACIT Fatigue Scores During 72 Weeks by Sex
Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Source: ADaM.ADQS

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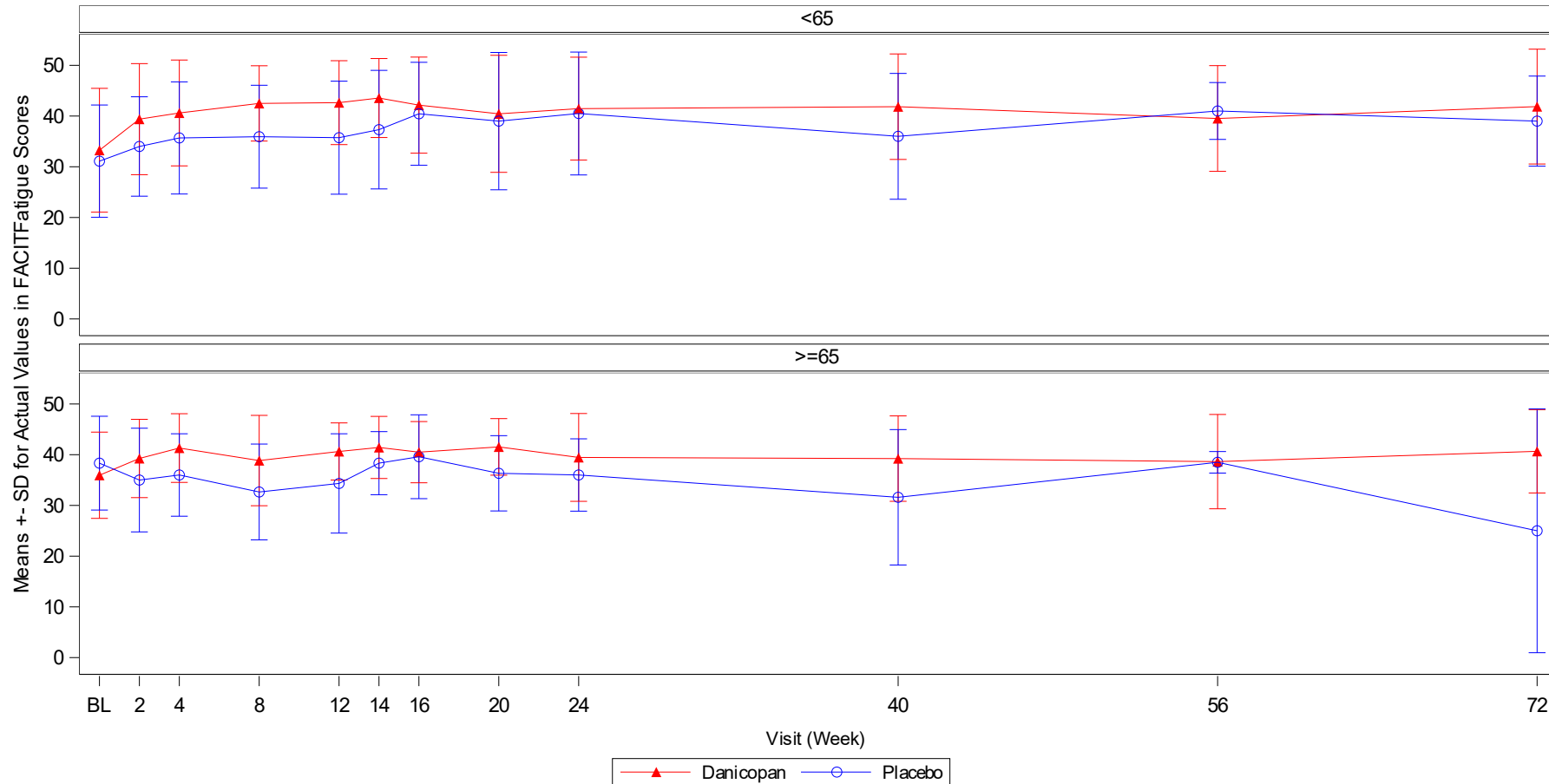
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Figure GERMANY.2.2.1.3.2
Plots of Mean \pm SD for Actual Values in FACIT Fatigue Scores During 72 Weeks by Age at Informed Consent
(< 65 and ≥ 65 Years)
Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Source: ADaM.ADQS

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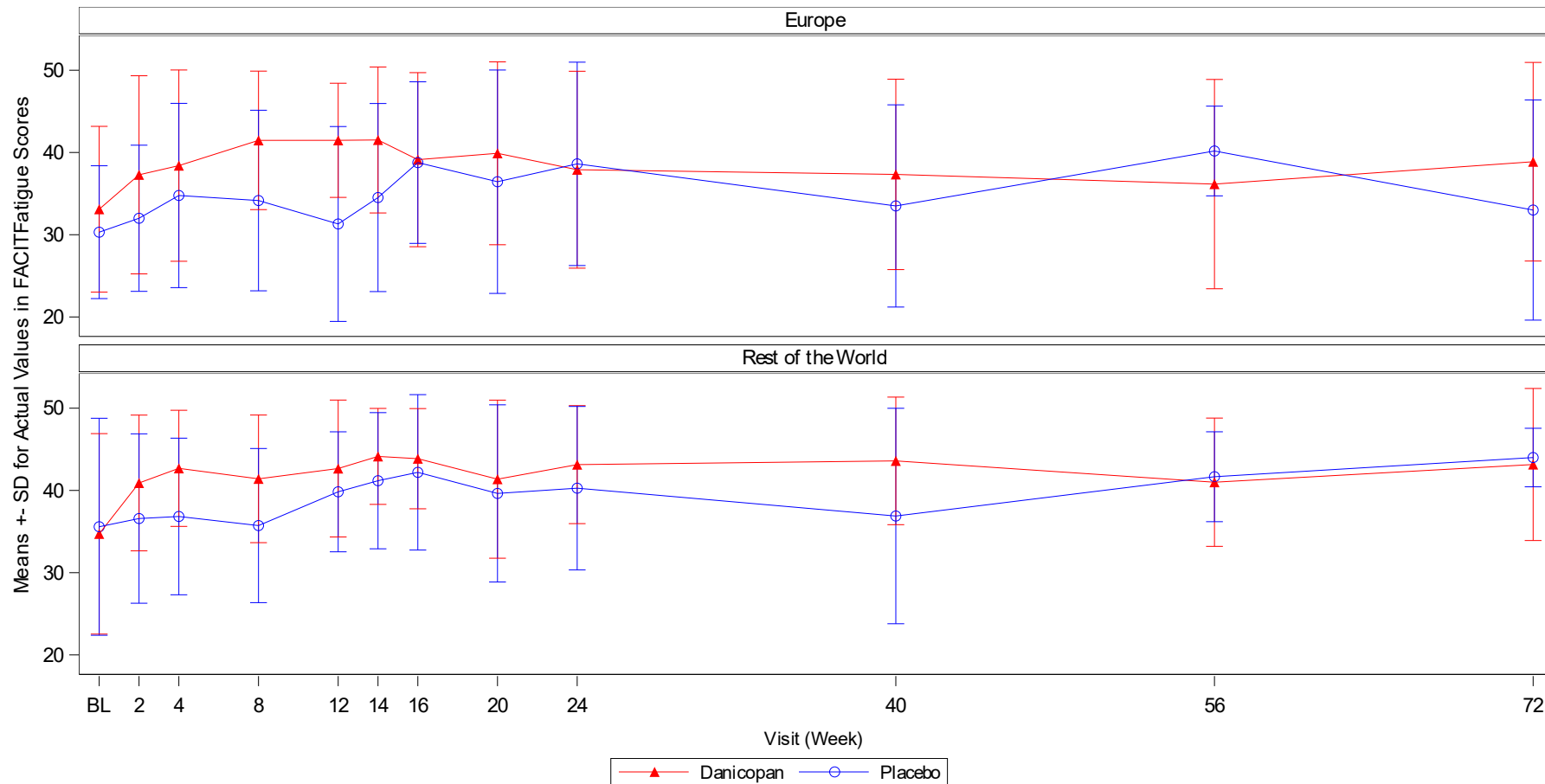
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Figure GERMANY.2.2.1.3.3
Plots of Mean \pm SD for Actual Values in FACIT Fatigue Scores During 72 Weeks by Region
Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Source: ADaM.ADQS

Run Date: 2024-04-26T15:30:41

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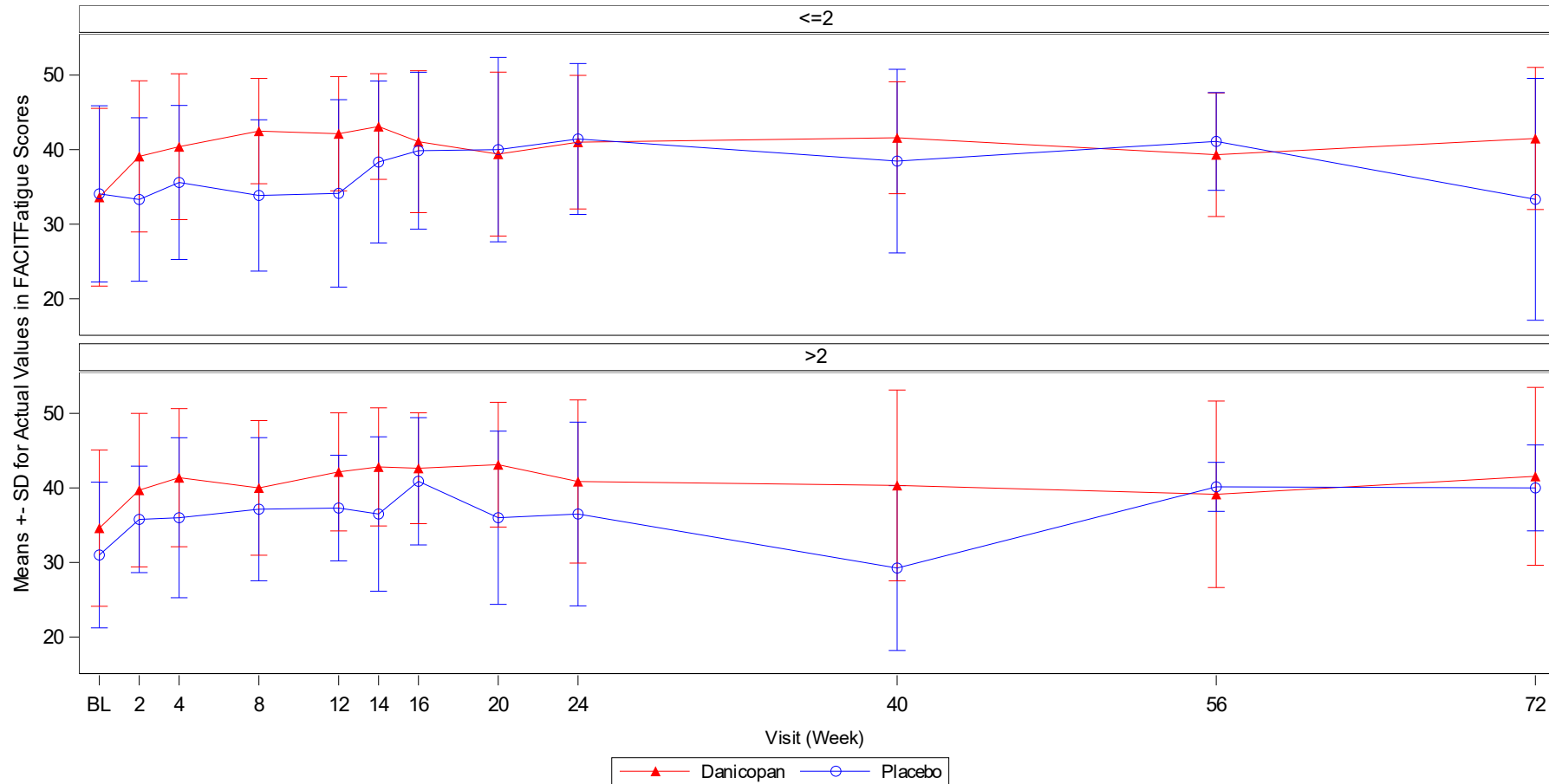
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Figure GERMANY.2.2.1.3.4

Plots of Mean \pm SD for Actual Values in FACIT Fatigue Scores During 72 Weeks by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Source: ADaM.ADQS

Run Date: 2024-04-26T15:30:41

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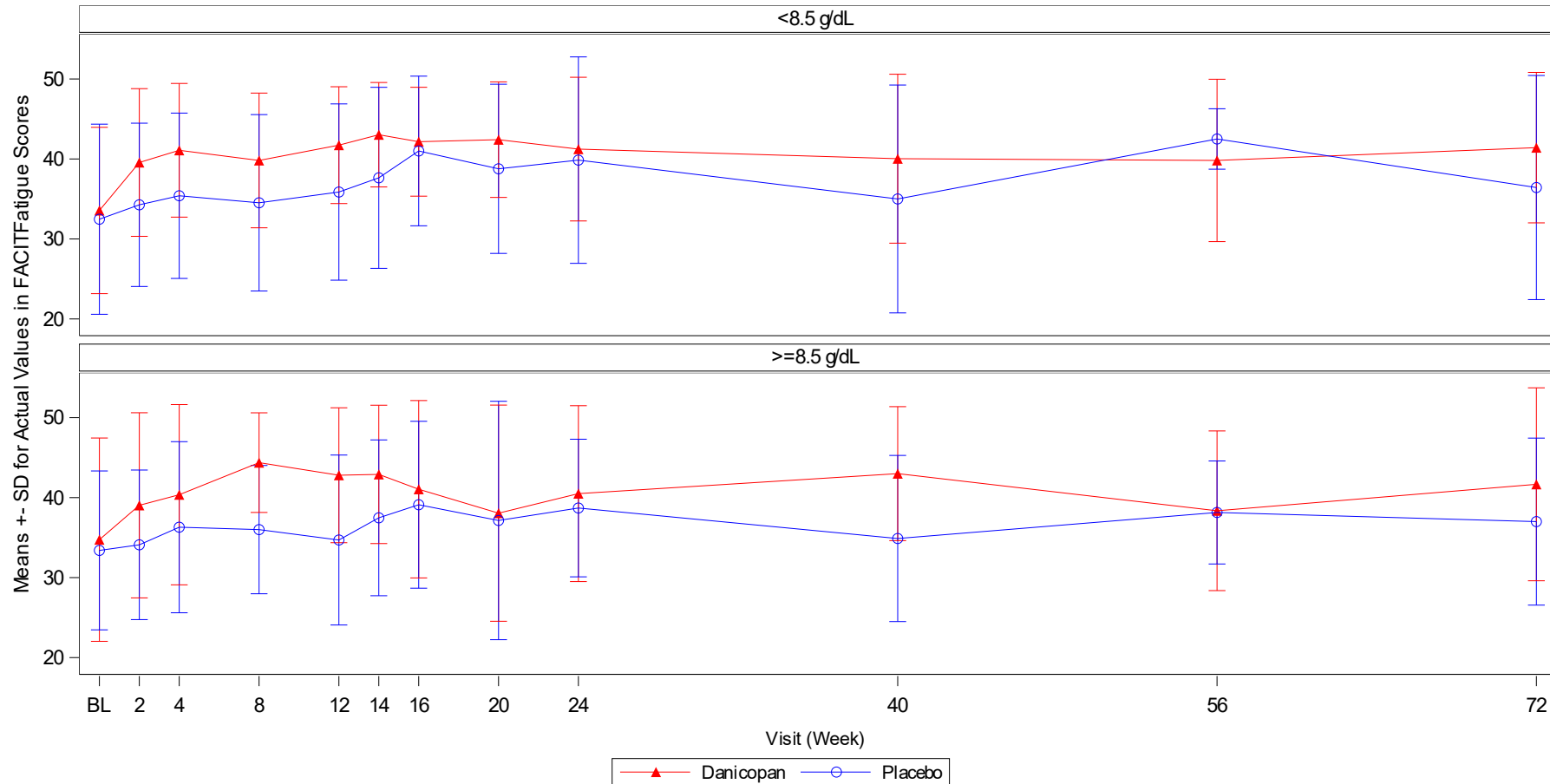
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Figure GERMANY.2.2.1.3.5

Plots of Mean \pm SD for Actual Values in FACIT Fatigue Scores During 72 Weeks by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Source: ADaM.ADQS

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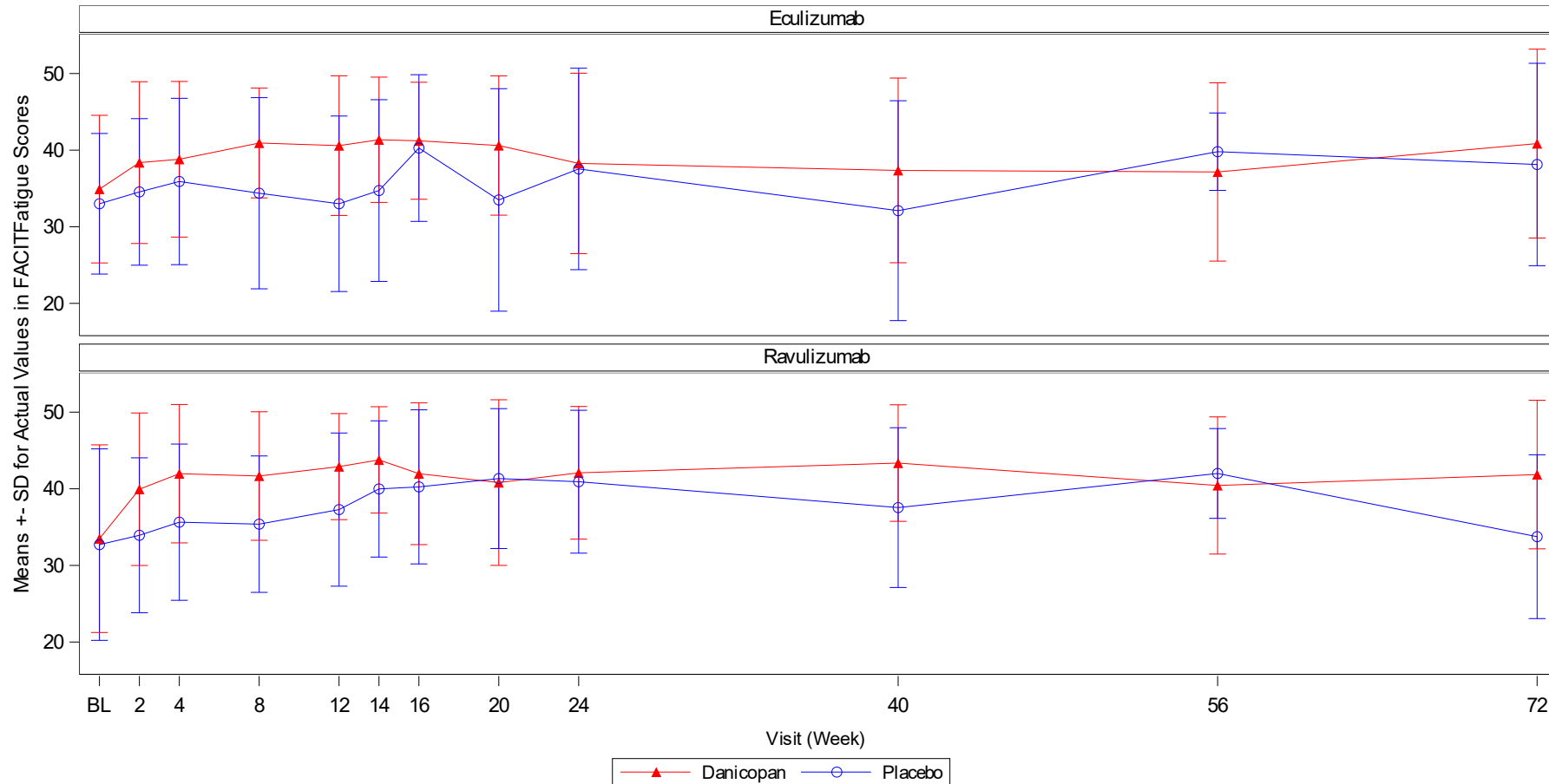
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Figure GERMANY.2.2.1.3.6
Plots of Mean \pm SD for Actual Values in FACIT Fatigue Scores During 72 Weeks by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Source: ADaM.ADQS

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Data cutoff date of 31Mar2023

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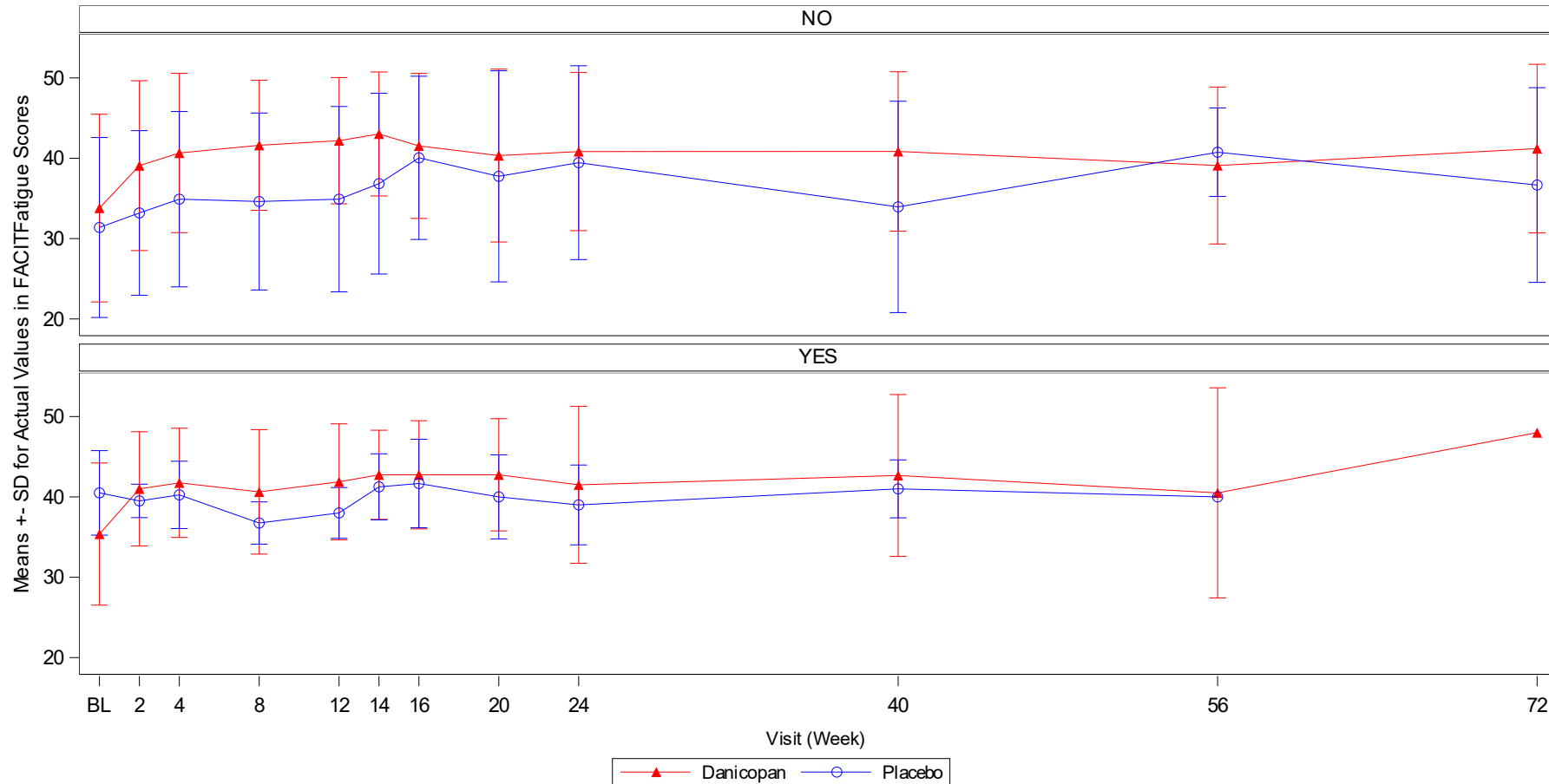
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Figure GERMANY.2.2.1.3.7

Plots of Mean \pm SD for Actual Values in FACIT Fatigue Scores During 72 Weeks by the Randomization Stratification of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Source: ADaM.ADQS

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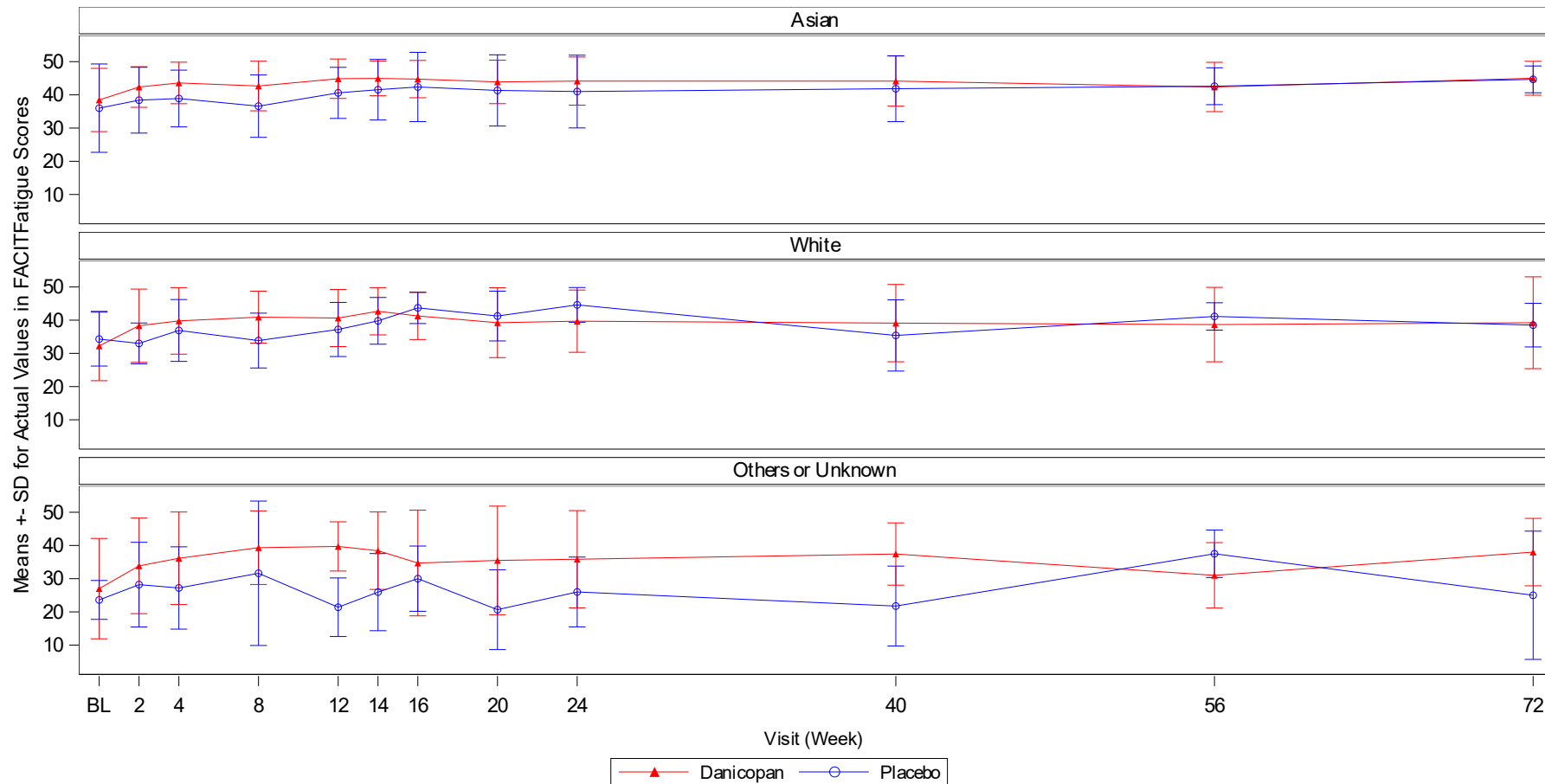
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Figure GERMANY.2.2.1.3.8
Plots of Mean \pm SD for Actual Values in FACIT Fatigue Scores During 72 Weeks by Race
Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Source: ADaM.ADQS

Run Date: 2024-04-26T15:30:42

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.3.1.1
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Male

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	4.68 (1.532)	1.16 (2.373)	3.52 (2.773)	0.2157
	95% CI for LS MEAN	(1.53, 7.83)	(-3.71, 6.04)	(-2.19, 9.23)	
Week 4	LS Mean (SEM)	5.92 (1.654)	2.38 (2.521)	3.53 (2.976)	0.2460
	95% CI for LS MEAN	(2.52, 9.32)	(-2.80, 7.57)	(-2.59, 9.66)	
Week 8	LS Mean (SEM)	5.28 (1.297)	1.21 (1.961)	4.07 (2.294)	0.0903
	95% CI for LS MEAN	(2.59, 7.98)	(-2.86, 5.28)	(-0.70, 8.85)	
Week 12	LS Mean (SEM)	5.93 (1.391)	1.83 (2.088)	4.10 (2.460)	0.1069
	95% CI for LS MEAN	(3.08, 8.78)	(-2.45, 6.11)	(-0.94, 9.15)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:53

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.3.1.1
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 16)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.44 (1.201)	1.21 (1.729)	5.23 (2.089)	0.0160
	95% CI for LS MEAN	(4.02, 8.86)	(-2.28, 4.69)	(1.02, 9.44)	
Week 4	LS Mean (SEM)	8.12 (1.392)	2.96 (2.025)	5.17 (2.441)	0.0396
	95% CI for LS MEAN	(5.33, 10.92)	(-1.11, 7.03)	(0.26, 10.07)	
Week 8	LS Mean (SEM)	9.14 (1.681)	-1.09 (2.457)	10.24 (2.963)	0.0013
	95% CI for LS MEAN	(5.75, 12.54)	(-6.05, 3.87)	(4.25, 16.22)	
Week 12	LS Mean (SEM)	9.65 (1.230)	2.71 (1.710)	6.94 (2.086)	0.0019
	95% CI for LS MEAN	(7.17, 12.13)	(-0.75, 6.16)	(2.73, 11.16)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Data cutoff date of 31Mar2023

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Table GERMANY.2.2.3.1.1
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.3170
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.2.3.1.2
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Age at Informed Consent
(< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

< 65 Years

Visit	Statistics	Danicopan (N = 40)	Placebo (N = 19)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.60 (1.237)	2.22 (1.770)	4.38 (2.125)	0.0439
	95% CI for LS MEAN	(4.12, 9.07)	(-1.33, 5.76)	(0.12, 8.64)	
Week 4	LS Mean (SEM)	7.90 (1.362)	3.90 (1.955)	4.00 (2.351)	0.0947
	95% CI for LS MEAN	(5.17, 10.62)	(-0.01, 7.81)	(-0.71, 8.71)	
Week 8	LS Mean (SEM)	9.16 (1.412)	0.75 (2.062)	8.42 (2.469)	0.0016
	95% CI for LS MEAN	(6.30, 12.03)	(-3.43, 4.93)	(3.41, 13.43)	
Week 12	LS Mean (SEM)	9.60 (1.088)	3.95 (1.528)	5.65 (1.835)	0.0033
	95% CI for LS MEAN	(7.42, 11.78)	(0.89, 7.02)	(1.97, 9.32)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:53

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Table GERMANY.2.2.3.1.2
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Age at Informed Consent
(< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	3.48 (0.998)	-1.95 (1.669)	5.43 (1.934)	0.0133
	95% CI for LS MEAN	(1.34, 5.61)	(-5.50, 1.60)	(1.31, 9.55)	
Week 4	LS Mean (SEM)	5.44 (1.720)	-1.79 (2.768)	7.23 (3.261)	0.0411
	95% CI for LS MEAN	(1.81, 9.08)	(-7.65, 4.08)	(0.33, 14.13)	
Week 8	LS Mean (SEM)	2.43 (1.713)	-4.99 (2.830)	7.43 (3.307)	0.0381
	95% CI for LS MEAN	(-1.17, 6.04)	(-10.96, 0.97)	(0.46, 14.39)	
Week 12	LS Mean (SEM)	4.45 (1.529)	-3.45 (2.292)	7.90 (2.760)	0.0122
	95% CI for LS MEAN	(1.20, 7.69)	(-8.38, 1.47)	(2.00, 13.80)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.2.3.1.2
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Age at Informed Consent
(< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.6946
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.2.3.1.3
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Europe

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 13)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.12 (1.665)	1.43 (2.257)	3.70 (2.801)	0.1960
	95% CI for LS MEAN	(1.73, 8.51)	(-3.17, 6.02)	(-2.00, 9.40)	
Week 4	LS Mean (SEM)	6.00 (1.718)	4.20 (2.347)	1.81 (2.902)	0.5380
	95% CI for LS MEAN	(2.51, 9.49)	(-0.57, 8.96)	(-4.09, 7.70)	
Week 8	LS Mean (SEM)	7.90 (2.079)	-0.28 (2.928)	8.18 (3.580)	0.0296
	95% CI for LS MEAN	(3.65, 12.16)	(-6.25, 5.70)	(0.87, 15.50)	
Week 12	LS Mean (SEM)	8.70 (1.380)	0.73 (1.845)	7.97 (2.293)	0.0016
	95% CI for LS MEAN	(5.89, 11.52)	(-3.03, 4.50)	(3.29, 12.65)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.2.3.1.3
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.91 (1.050)	1.56 (1.708)	4.35 (1.949)	0.0317
	95% CI for LS MEAN	(3.78, 8.03)	(-1.89, 5.01)	(0.40, 8.29)	
Week 4	LS Mean (SEM)	7.69 (1.179)	1.57 (1.865)	6.12 (2.166)	0.0074
	95% CI for LS MEAN	(5.31, 10.07)	(-2.20, 5.34)	(1.74, 10.50)	
Week 8	LS Mean (SEM)	6.94 (1.294)	0.38 (1.983)	6.55 (2.325)	0.0078
	95% CI for LS MEAN	(4.32, 9.55)	(-3.63, 4.40)	(1.84, 11.27)	
Week 12	LS Mean (SEM)	7.21 (1.139)	4.57 (1.748)	2.64 (2.043)	0.2035
	95% CI for LS MEAN	(4.91, 9.51)	(1.03, 8.11)	(-1.49, 6.78)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.2.3.1.3
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.3152
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.2.3.1.4

Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.68 (1.733)	3.27 (2.635)	2.41 (3.074)	0.4397
	95% CI for LS MEAN	(2.14, 9.21)	(-2.10, 8.64)	(-3.87, 8.68)	
Week 4	LS Mean (SEM)	7.51 (1.727)	3.53 (2.533)	3.98 (2.992)	0.1942
	95% CI for LS MEAN	(3.99, 11.03)	(-1.65, 8.71)	(-2.15, 10.10)	
Week 8	LS Mean (SEM)	5.24 (1.957)	3.44 (2.952)	1.80 (3.476)	0.6086
	95% CI for LS MEAN	(1.21, 9.27)	(-2.64, 9.52)	(-5.37, 8.98)	
Week 12	LS Mean (SEM)	7.54 (1.350)	4.83 (1.919)	2.70 (2.258)	0.2416
	95% CI for LS MEAN	(4.77, 10.30)	(0.90, 8.77)	(-1.93, 7.34)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factor of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.2.3.1.4

Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.42 (1.090)	-0.74 (1.602)	6.17 (1.937)	0.0027
	95% CI for LS MEAN	(3.22, 7.62)	(-3.98, 2.49)	(2.26, 10.07)	
Week 4	LS Mean (SEM)	6.69 (1.402)	1.59 (2.080)	5.10 (2.508)	0.0481
	95% CI for LS MEAN	(3.86, 9.51)	(-2.60, 5.78)	(0.05, 10.15)	
Week 8	LS Mean (SEM)	8.55 (1.359)	-3.47 (1.998)	12.02 (2.416)	<0.0001
	95% CI for LS MEAN	(5.79, 11.31)	(-7.53, 0.58)	(7.12, 16.93)	
Week 12	LS Mean (SEM)	8.19 (1.236)	0.12 (1.779)	8.06 (2.166)	0.0006
	95% CI for LS MEAN	(5.69, 10.68)	(-3.47, 3.71)	(3.70, 12.43)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factor of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.2.3.1.4

Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.0449
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factor of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.2.3.1.5
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors
of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.43 (1.228)	1.45 (1.833)	4.99 (2.207)	0.0289
	95% CI for LS MEAN	(3.96, 8.90)	(-2.25, 5.14)	(0.54, 9.43)	
Week 4	LS Mean (SEM)	7.90 (1.250)	2.58 (1.883)	5.32 (2.261)	0.0229
	95% CI for LS MEAN	(5.38, 10.42)	(-1.21, 6.37)	(0.77, 9.88)	
Week 8	LS Mean (SEM)	6.31 (1.303)	0.28 (1.970)	6.03 (2.363)	0.0143
	95% CI for LS MEAN	(3.68, 8.94)	(-3.69, 4.25)	(1.27, 10.79)	
Week 12	LS Mean (SEM)	7.86 (1.204)	3.05 (1.798)	4.81 (2.165)	0.0314
	95% CI for LS MEAN	(5.43, 10.28)	(-0.58, 6.67)	(0.45, 9.17)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.2.3.1.5
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors
of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.14 (1.580)	1.20 (2.362)	3.94 (2.741)	0.1637
	95% CI for LS MEAN	(1.89, 8.39)	(-3.65, 6.06)	(-1.72, 9.60)	
Week 4	LS Mean (SEM)	6.47 (1.893)	3.09 (2.738)	3.38 (3.255)	0.3079
	95% CI for LS MEAN	(2.61, 10.32)	(-2.50, 8.68)	(-3.27, 10.03)	
Week 8	LS Mean (SEM)	10.17 (2.124)	0.57 (3.138)	9.60 (3.722)	0.0206
	95% CI for LS MEAN	(5.68, 14.66)	(-6.09, 7.22)	(1.69, 17.52)	
Week 12	LS Mean (SEM)	8.71 (1.448)	1.49 (1.982)	7.22 (2.362)	0.0050
	95% CI for LS MEAN	(5.74, 11.67)	(-2.58, 5.56)	(2.37, 12.06)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.2.3.1.5
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors
of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.7590
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factor of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.2.3.1.6
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Background C5 Inhibitor Therapy
(Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 11)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	3.96 (1.722)	1.83 (2.435)	2.13 (2.936)	0.4733
	95% CI for LS MEAN	(0.43, 7.50)	(-3.15, 6.81)	(-3.89, 8.16)	
Week 4	LS Mean (SEM)	4.40 (1.907)	2.72 (2.606)	1.68 (3.192)	0.6017
	95% CI for LS MEAN	(0.50, 8.30)	(-2.61, 8.04)	(-4.85, 8.22)	
Week 8	LS Mean (SEM)	4.22 (1.800)	-0.83 (2.610)	5.05 (3.127)	0.1201
	95% CI for LS MEAN	(0.49, 7.95)	(-6.22, 4.55)	(-1.42, 11.52)	
Week 12	LS Mean (SEM)	6.23 (1.498)	-0.19 (1.888)	6.43 (2.360)	0.0113
	95% CI for LS MEAN	(3.16, 9.30)	(-4.08, 3.69)	(1.58, 11.27)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.2.3.1.6
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Background C5 Inhibitor Therapy
(Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.61 (1.121)	0.82 (1.737)	5.79 (2.039)	0.0069
	95% CI for LS MEAN	(4.35, 8.87)	(-2.68, 4.32)	(1.67, 9.90)	
Week 4	LS Mean (SEM)	8.69 (1.234)	2.54 (1.929)	6.15 (2.264)	0.0092
	95% CI for LS MEAN	(6.21, 11.17)	(-1.35, 6.42)	(1.59, 10.71)	
Week 8	LS Mean (SEM)	8.85 (1.420)	0.28 (2.177)	8.57 (2.574)	0.0020
	95% CI for LS MEAN	(5.98, 11.73)	(-4.13, 4.69)	(3.35, 13.80)	
Week 12	LS Mean (SEM)	9.04 (1.077)	4.18 (1.666)	4.87 (1.953)	0.0170
	95% CI for LS MEAN	(6.87, 11.22)	(0.81, 7.55)	(0.92, 8.81)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.2.3.1.6
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Background C5 Inhibitor Therapy
(Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.8296
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.2.3.1.7
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors
of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	7.91 (5.541)	-0.30 (3.110)	8.21 (7.574)	0.3116
	95% CI for LS MEAN	(-5.05, 20.87)	(-7.54, 6.93)	(-9.44, 25.86)	
Week 4	LS Mean (SEM)	8.66 (5.797)	0.45 (3.934)	8.21 (8.129)	0.3364
	95% CI for LS MEAN	(-4.39, 21.71)	(-8.57, 9.46)	(-9.93, 26.36)	
Week 8	LS Mean (SEM)	7.53 (5.781)	-3.05 (3.888)	10.59 (8.095)	0.2207
	95% CI for LS MEAN	(-5.50, 20.57)	(-11.93, 5.82)	(-7.49, 28.67)	
Week 12	LS Mean (SEM)	8.78 (5.728)	-1.80 (3.726)	10.59 (7.980)	0.2170
	95% CI for LS MEAN	(-4.24, 21.81)	(-10.54, 6.93)	(-7.44, 28.62)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.2.3.1.7
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 48)	Placebo (N = 21)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.93 (1.102)	1.25 (1.649)	4.68 (1.959)	0.0199
	95% CI for LS MEAN	(3.73, 8.13)	(-2.04, 4.54)	(0.77, 8.59)	
Week 4	LS Mean (SEM)	7.51 (1.198)	2.87 (1.778)	4.65 (2.123)	0.0321
	95% CI for LS MEAN	(5.12, 9.91)	(-0.68, 6.42)	(0.41, 8.88)	
Week 8	LS Mean (SEM)	8.11 (1.337)	-0.24 (2.014)	8.36 (2.397)	0.0010
	95% CI for LS MEAN	(5.43, 10.79)	(-4.28, 3.80)	(3.54, 13.17)	
Week 12	LS Mean (SEM)	8.55 (1.023)	2.87 (1.461)	5.68 (1.757)	0.0020
	95% CI for LS MEAN	(6.51, 10.59)	(-0.05, 5.79)	(2.17, 9.20)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.2.3.1.7
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors
of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.9101
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.2.3.1.8
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	4.09 (1.225)	1.34 (1.706)	2.75 (1.958)	0.1720
	95% CI for LS MEAN	(1.58, 6.59)	(-2.16, 4.84)	(-1.27, 6.77)	
Week 4	LS Mean (SEM)	5.31 (1.421)	1.84 (2.013)	3.48 (2.344)	0.1494
	95% CI for LS MEAN	(2.41, 8.21)	(-2.28, 5.95)	(-1.33, 8.28)	
Week 8	LS Mean (SEM)	4.98 (1.572)	-0.46 (2.197)	5.44 (2.589)	0.0445
	95% CI for LS MEAN	(1.78, 8.18)	(-4.95, 4.03)	(0.14, 10.74)	
Week 12	LS Mean (SEM)	5.81 (1.457)	3.54 (2.033)	2.27 (2.384)	0.3497
	95% CI for LS MEAN	(2.83, 8.80)	(-0.65, 7.72)	(-2.64, 7.19)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.2.3.1.8
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

White

Visit	Statistics	Danicopan (N = 27)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.29 (1.272)	-0.63 (2.155)	6.92 (2.495)	0.0093
	95% CI for LS MEAN	(3.69, 8.89)	(-5.02, 3.76)	(1.83, 12.01)	
Week 4	LS Mean (SEM)	7.79 (1.527)	3.12 (2.520)	4.67 (2.942)	0.1214
	95% CI for LS MEAN	(4.69, 10.90)	(-2.00, 8.24)	(-1.31, 10.65)	
Week 8	LS Mean (SEM)	7.63 (1.791)	-1.22 (3.040)	8.85 (3.526)	0.0188
	95% CI for LS MEAN	(3.94, 11.33)	(-7.47, 5.04)	(1.59, 16.11)	
Week 12	LS Mean (SEM)	8.27 (1.221)	3.42 (1.947)	4.85 (2.295)	0.0434
	95% CI for LS MEAN	(5.77, 10.76)	(-0.57, 7.41)	(0.15, 9.54)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.2.3.1.8
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 5)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	10.86 (4.415)	4.92 (4.664)	5.94 (6.145)	0.3496
	95% CI for LS MEAN	(1.19, 20.53)	(-5.04, 14.88)	(-7.20, 19.08)	
Week 4	LS Mean (SEM)	13.15 (4.415)	3.92 (4.664)	9.23 (6.145)	0.1547
	95% CI for LS MEAN	(3.48, 22.81)	(-6.04, 13.88)	(-3.91, 22.36)	
Week 8	LS Mean (SEM)	17.59 (5.803)	5.43 (9.302)	12.16 (10.877)	0.2755
	95% CI for LS MEAN	(5.42, 29.77)	(-13.98, 24.85)	(-10.38, 34.70)	
Week 12	LS Mean (SEM)	16.72 (4.415)	-1.88 (4.664)	18.60 (6.145)	0.0088
	95% CI for LS MEAN	(7.05, 26.38)	(-11.84, 8.08)	(5.46, 31.74)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.2.3.1.8
Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.0477
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline FACIT Fatigue scores value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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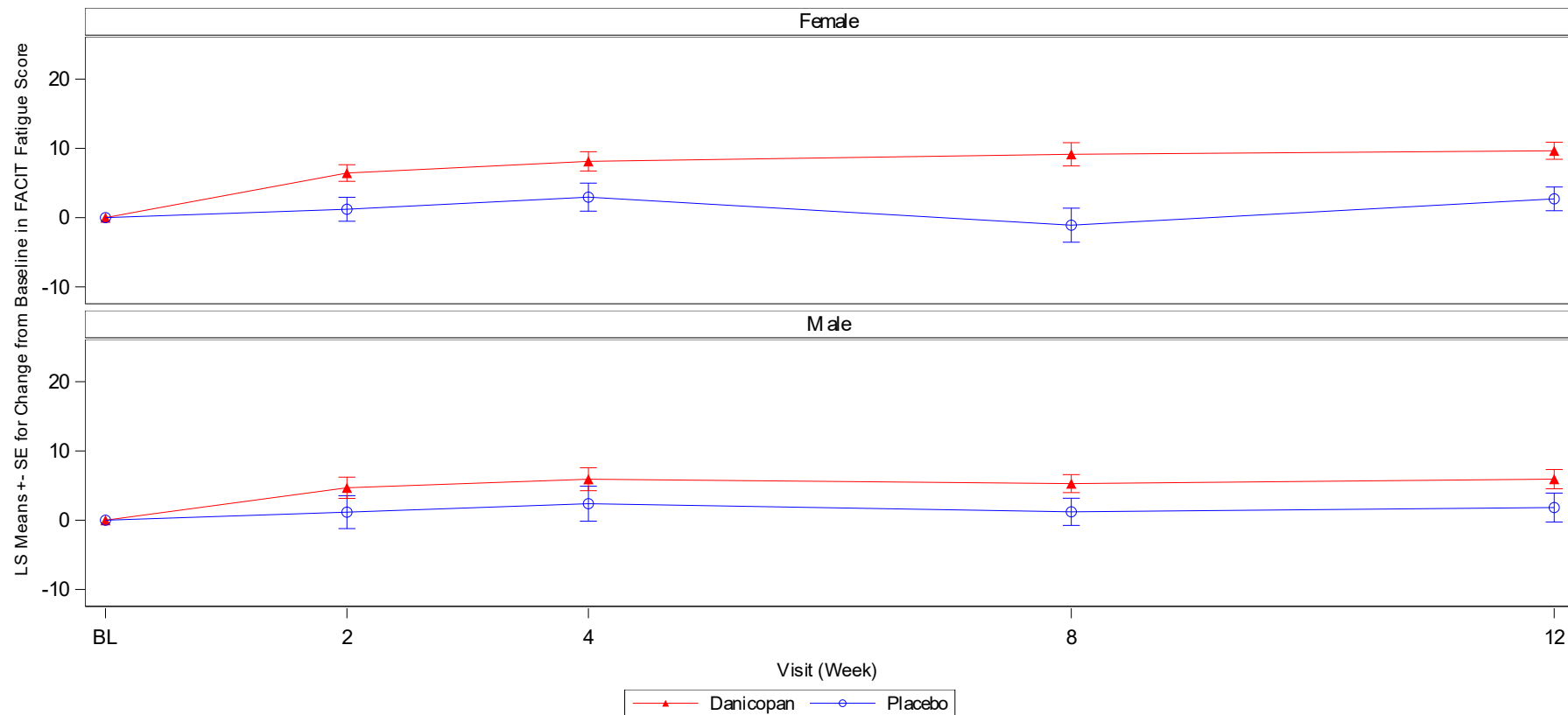
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Figure GERMANY.2.2.1.1.1

Plots of LS Means \pm SE for Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Sex
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).
FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
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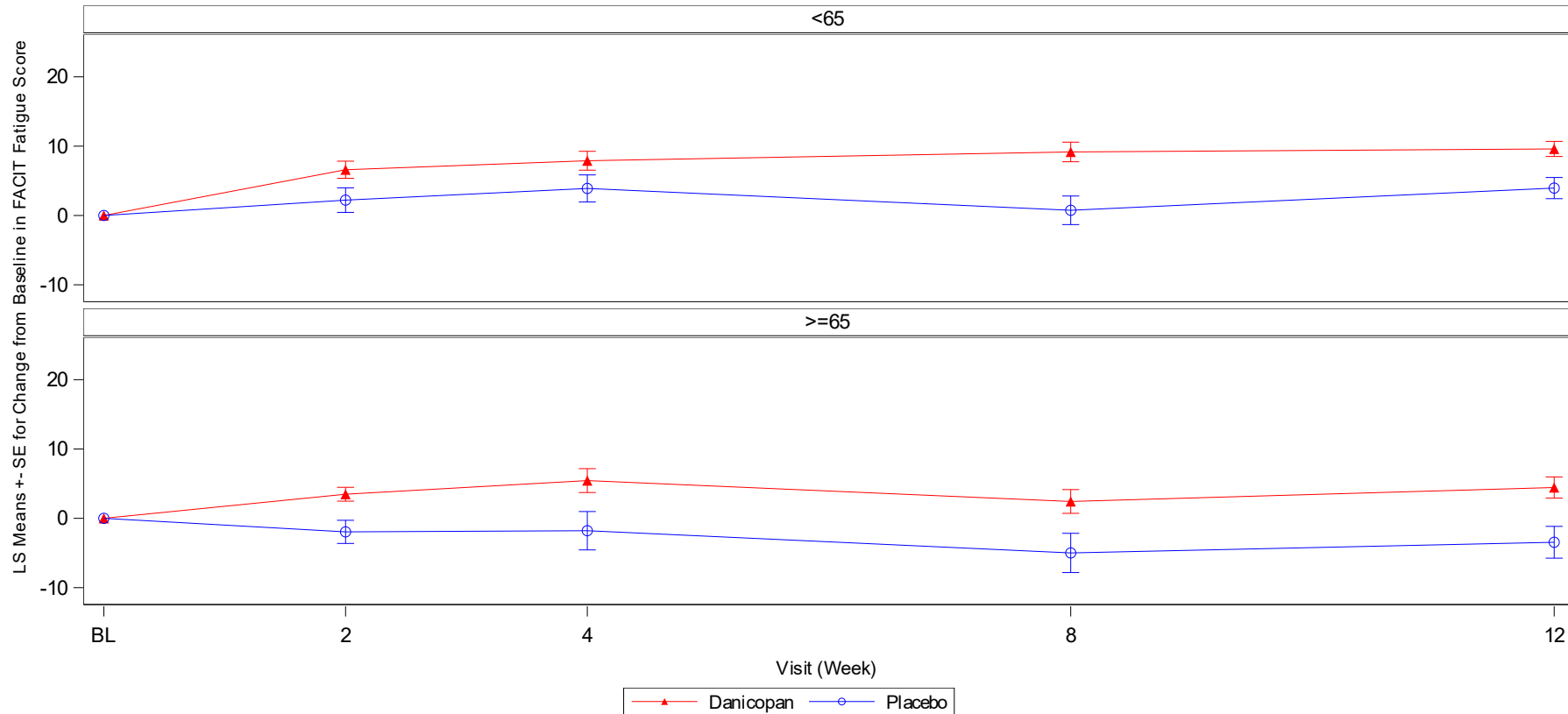
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Figure GERMANY.2.2.1.1.2
Plots of LS Means +- SE for Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).
FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
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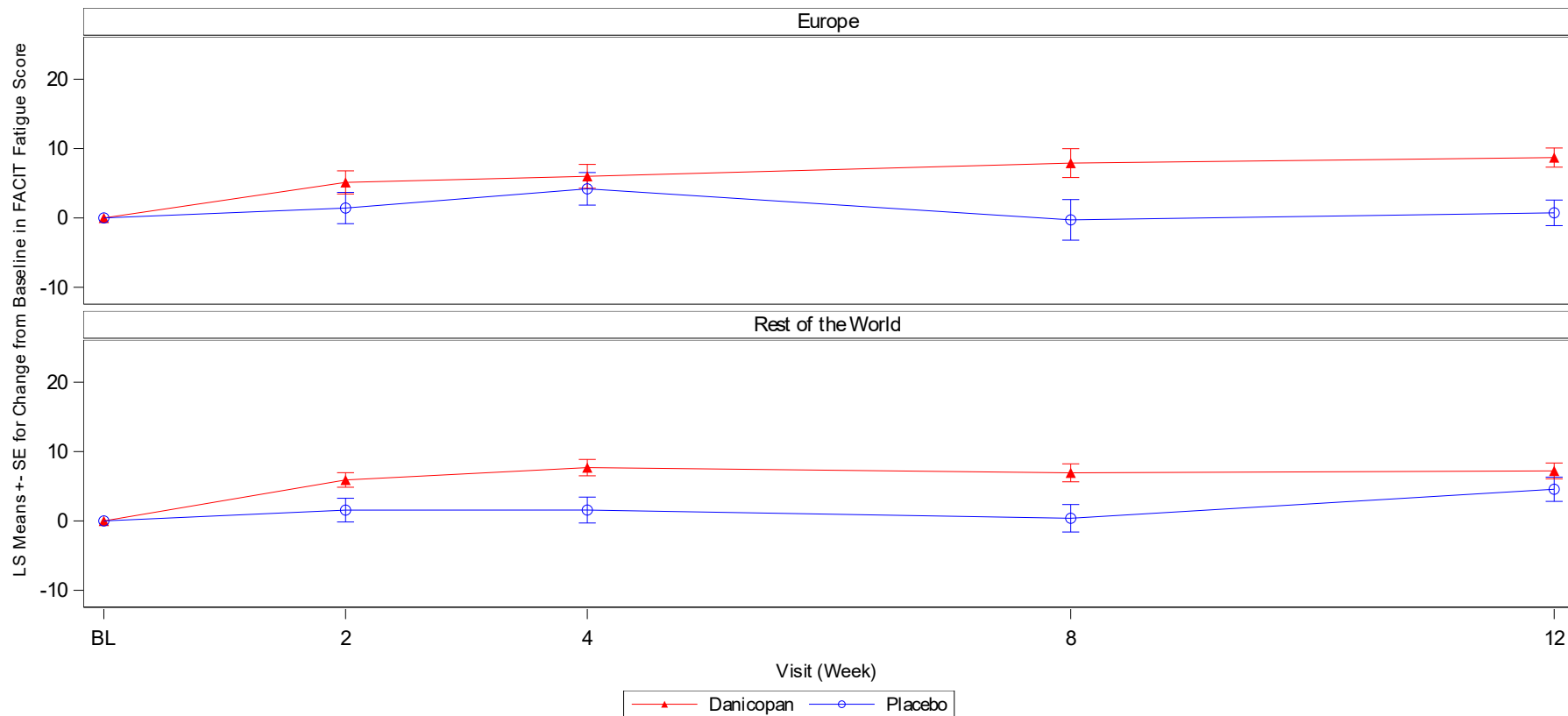
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Figure GERMANY.2.2.1.1.3

Plots of LS Means +- SE for Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Region
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Source: ADaM.ADSL, ADaM.ADQS

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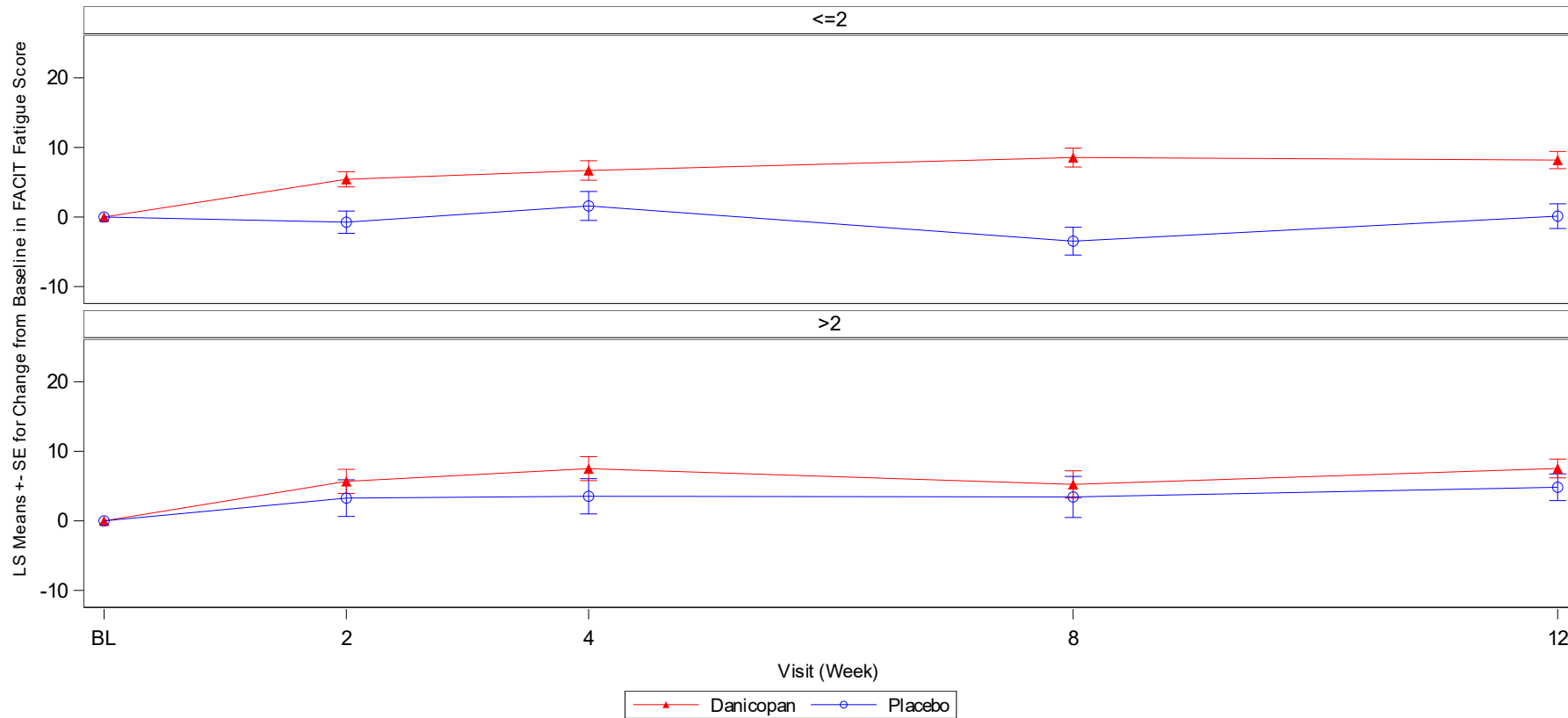
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Figure GERMANY.2.2.1.1.4
Plots of LS Means +/- SE for Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Transfusion History
(> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set



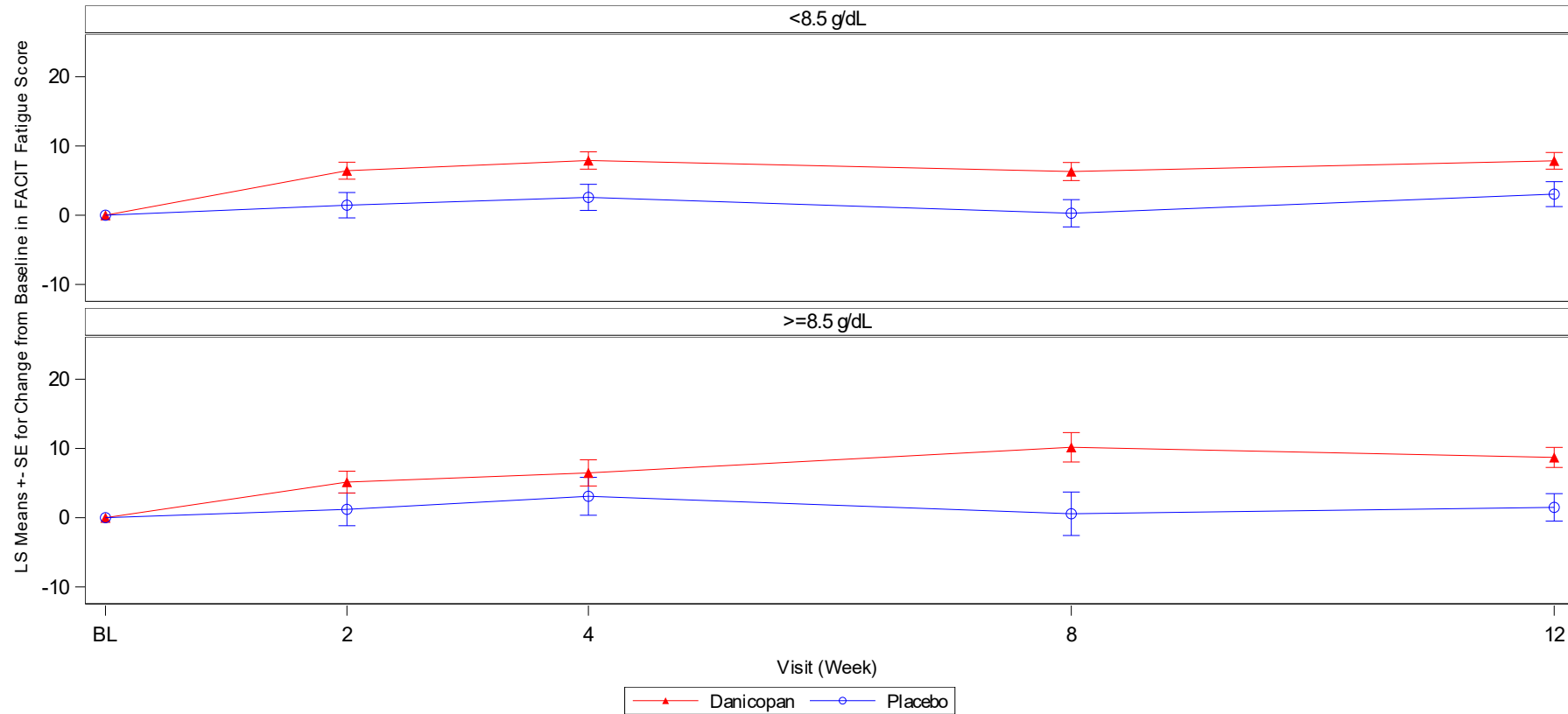
LS=Least squares; SE=Standard error of the mean.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).
FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
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Figure GERMANY.2.2.1.1.5
Plots of LS Means \pm SE for Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).
FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
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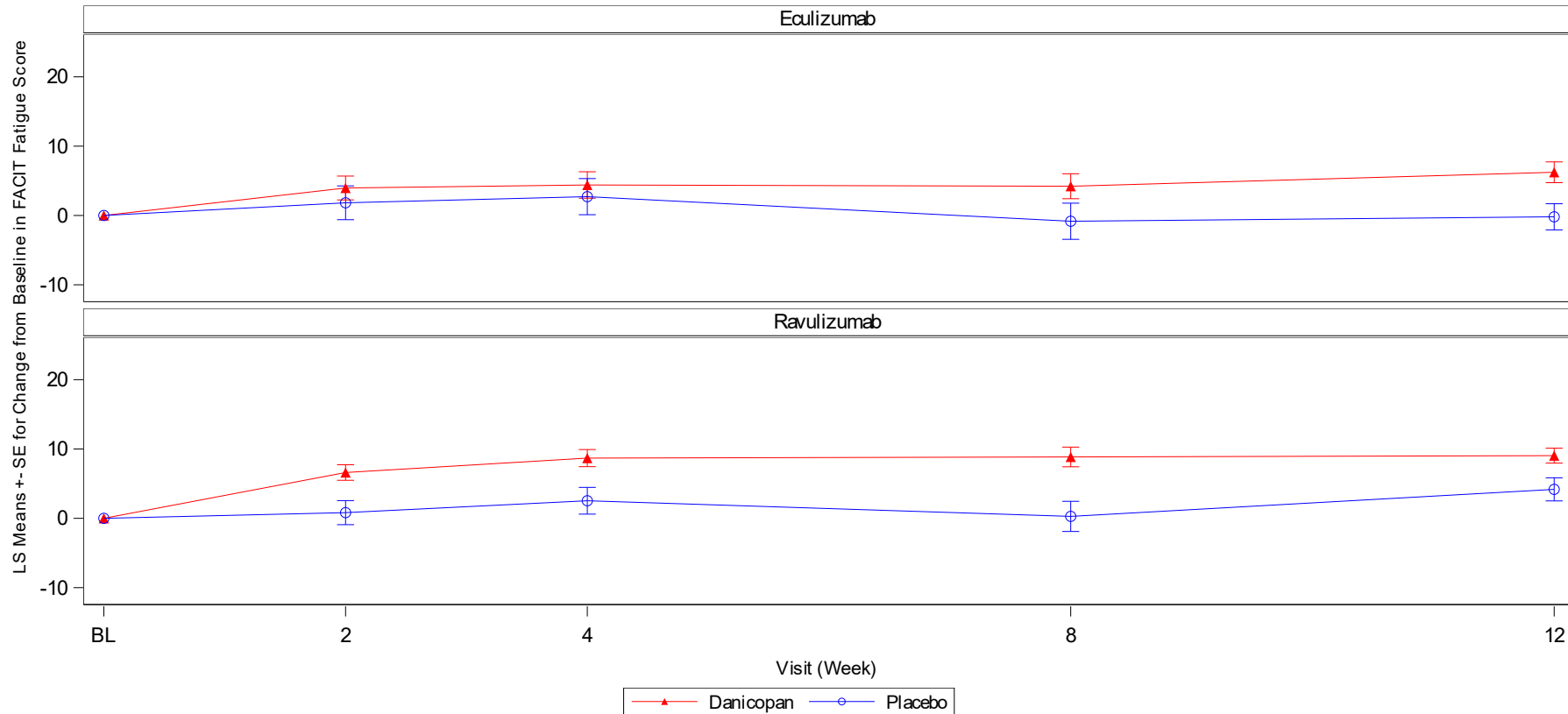
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Figure GERMANY.2.2.1.1.6
Plots of LS Means \pm SE for Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.

Source: ADaM.ADSL, ADaM.ADQS

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Data cutoff date of 31Mar2023

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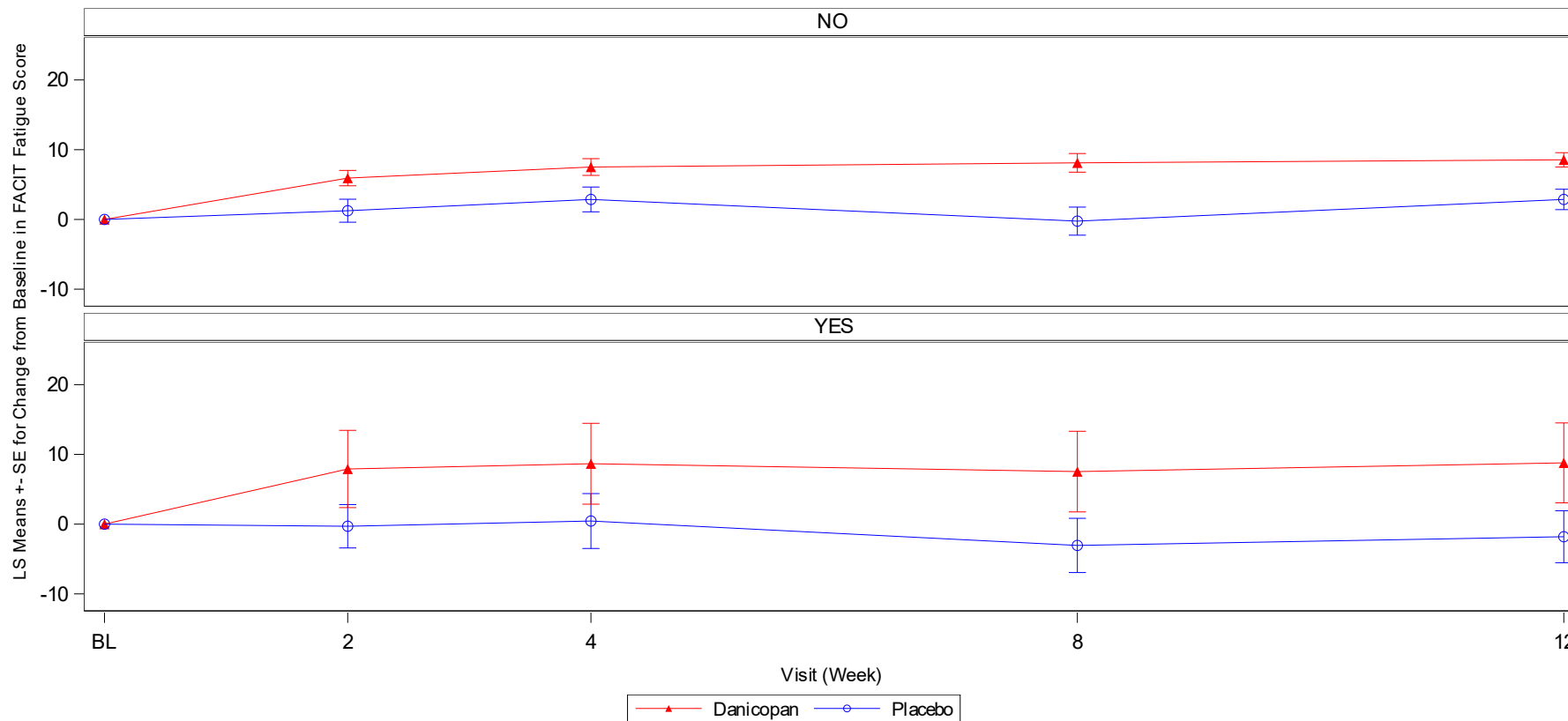
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Figure GERMANY.2.2.1.1.7

Plots of LS Means \pm SE for Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).
FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Source: ADaM.ADSL, ADaM.ADQS
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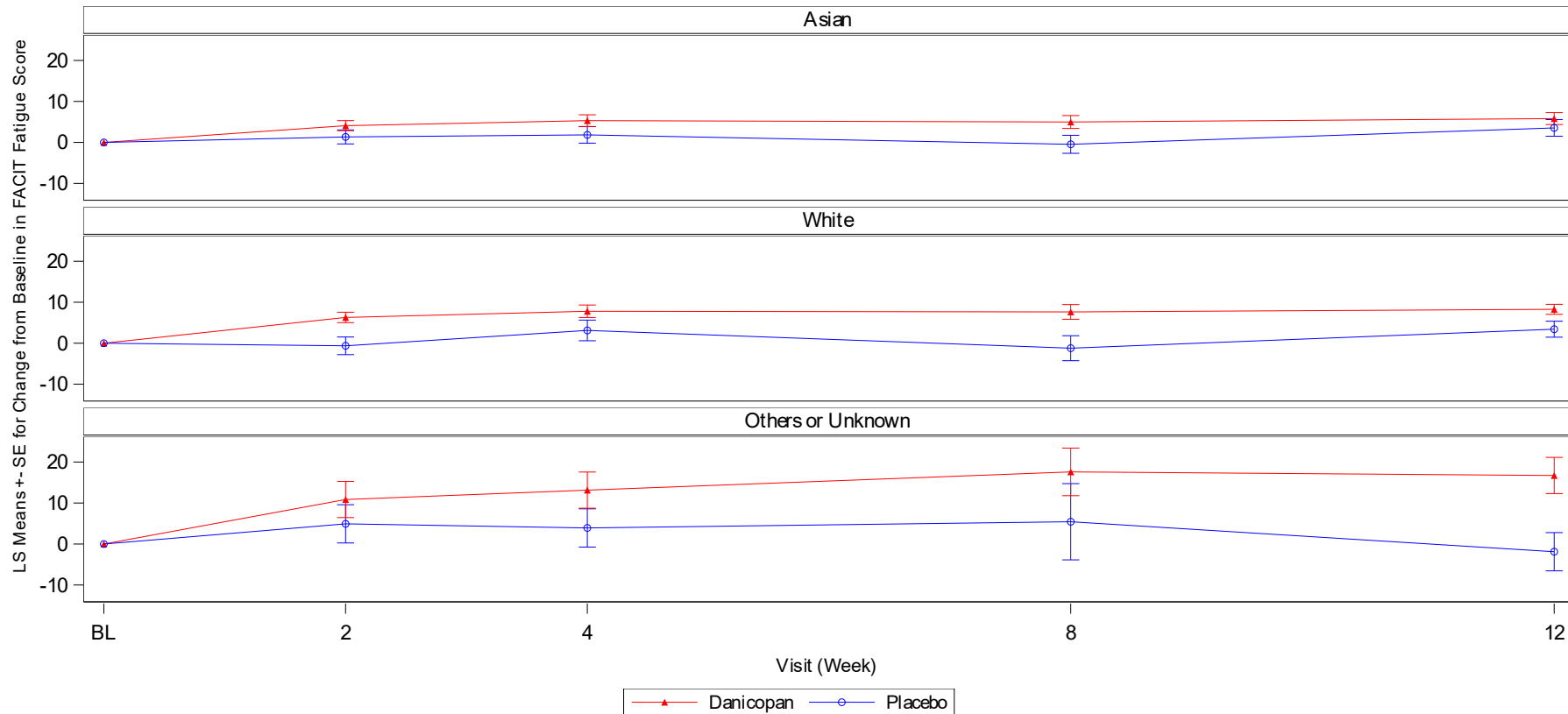
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Figure GERMANY.2.2.1.1.8

Plots of LS Means \pm SE for Change from Baseline in FACIT Fatigue Scores During 12-Week of Treatment Period 1 by Race
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).
FACIT Fatigue score ranges from 0-52, with a higher score indicating less fatigue.
Source: ADaM.ADSL, ADaM.ADQS
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Data cutoff date of 31Mar2023
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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.6 Symptomatik anhand des EORTC QLQ-C30

1.6.1 Rücklaufquoten des EORTC QLQ-C30

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Table GERMANY.2.5.4.10
EORTC QLQ-C30 Scores Compliance Rate During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit	Statistics	D->D (N=57)	P->D (N=26)
Baseline	Expected Patients Per Visit	57	26
	Patients Per Visit	57	26
	Patients with at Least One Completed Question, n (%)	57 (100)	24 (92.3)
	Patients with Fully Completed Questionnaire, n (%)	57 (100)	24 (92.3)
Week 2	Expected Patients Per Visit	57	26
	Patients Per Visit	57	26
	Patients with at Least One Completed Question, n (%)	57 (100)	25 (96.2)
	Patients with Fully Completed Questionnaire, n (%)	55 (96.5)	23 (88.5)
Week 4	Expected Patients Per Visit	57	25
	Patients Per Visit	57	25
	Patients with at Least One Completed Question, n (%)	57 (100)	25 (100)
	Patients with Fully Completed Questionnaire, n (%)	56 (98.2)	24 (96.0)
Week 8	Expected Patients Per Visit	57	25
	Patients Per Visit	57	25
	Patients with at Least One Completed Question, n (%)	45 (78.9)	19 (76.0)
	Patients with Fully Completed Questionnaire, n (%)	44 (77.2)	18 (72.0)

Percentages are based on the total number of expected patients in each group.

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem. (0= Not at all, 1 = A little bit, 2 = Somewhat, 3 = Quite a bit, 4 = Very much)

Subscale Score: PF2=Physical functioning, RF2=Role functioning, EF=Emotional functioning, CF=Cognitive functioning, SF=Social functioning, FA=Fatigue, NV=Nausea and vomiting, PA=Pain, DY=Dyspnoea, SL=Insomnia, AP=Appetite loss, CO=Constipation, DI=Diarrhoea, FI=Financial difficulties, QL2=Global health status/QoL

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.10
EORTC QLQ-C30 Scores Compliance Rate During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit	Statistics	D->D (N=57)	P->D (N=26)
Week 12	Expected Patients Per Visit	55	25
	Patients Per Visit	54	25
	Patients with at Least One Completed Question, n (%)	52 (94.5)	25 (100)
	Patients with Fully Completed Questionnaire, n (%)	52 (94.5)	24 (96.0)
Week 24	Expected Patients Per Visit	55	24
	Patients Per Visit	55	24
	Patients with at Least One Completed Question, n (%)	49 (89.1)	24 (100)
	Patients with Fully Completed Questionnaire, n (%)	49 (89.1)	23 (95.8)
Week 40	Expected Patients Per Visit	46	22
	Patients Per Visit	46	22
	Patients with at Least One Completed Question, n (%)	46 (100)	21 (95.5)
	Patients with Fully Completed Questionnaire, n (%)	46 (100)	21 (95.5)
Week 56	Expected Patients Per Visit	36	18
	Patients Per Visit	36	18
	Patients with at Least One Completed Question, n (%)	34 (94.4)	17 (94.4)
	Patients with Fully Completed Questionnaire, n (%)	34 (94.4)	17 (94.4)

Percentages are based on the total number of expected patients in each group.

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem. (0= Not at all, 1 = A little bit, 2 = Somewhat, 3 = Quite a bit, 4 = Very much)

Subscale Score: PF2=Physical functioning, RF2=Role functioning, EF=Emotional functioning, CF=Cognitive functioning, SF=Social functioning, FA=Fatigue, NV=Nausea and vomiting, PA=Pain, DY=Dyspnoea, SL=Insomnia, AP=Appetite loss, CO=Constipation, DI=Diarrhoea, FI=Financial difficulties, QL2=Global health status/QoL

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.10
EORTC QLQ-C30 Scores Compliance Rate During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit	Statistics	D->D (N=57)	P->D (N=26)
Week 72	Expected Patients Per Visit	21	12
	Patients Per Visit	21	12
	Patients with at Least One Completed Question, n (%)	21 (100)	12 (100)
	Patients with Fully Completed Questionnaire, n (%)	20 (95.2)	9 (75.0)
Week 88	Expected Patients Per Visit	14	8
	Patients Per Visit	14	8
	Patients with at Least One Completed Question, n (%)	14 (100)	8 (100)
	Patients with Fully Completed Questionnaire, n (%)	14 (100)	8 (100)
Week 104	Expected Patients Per Visit	4	2
	Patients Per Visit	4	2
	Patients with at Least One Completed Question, n (%)	4 (100)	2 (100)
	Patients with Fully Completed Questionnaire, n (%)	4 (100)	2 (100)

Percentages are based on the total number of expected patients in each group.

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem. (0= Not at all, 1 = A little bit, 2 = Somewhat, 3 = Quite a bit, 4 = Very much)

Subscale Score: PF2=Physical functioning, RF2=Role functioning, EF=Emotional functioning, CF=Cognitive functioning, SF=Social functioning, FA=Fatigue, NV=Nausea and vomiting, PA=Pain, DY=Dyspnoea, SL=Insomnia, AP=Appetite loss, CO=Constipation, DI=Diarrhoea, FI=Financial difficulties, QL2=Global health status/QoL

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Source: ADaM.ADSL, ADaM.ADQS

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.6 Symptomatik anhand des EORTC QLQ-C30

1.6.2 Veränderung der EORTC QLQ-C30 Symptomskalen im Vergleich zu Baseline

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Fatigue

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	57		24		81	
Mean (SD)	43.27 (27.191)		48.15 (26.752)		44.72 (26.987)	
Median	33.33		44.44		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	55	55	25	23		
Mean (SD)	32.53 (25.558)	-11.11 (21.807)	44.89 (22.562)	-0.97 (22.200)		
Median	33.33	-11.11	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	11.1, 100.0	-44.4, 44.4		
Week 4						
n	57	57	25	24		
Mean (SD)	27.97 (23.049)	-15.30 (24.040)	46.67 (23.130)	0.00 (23.627)		
Median	33.33	-11.11	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 44.4	11.1, 100.0	-66.7, 44.4		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Fatigue

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	45	45	19	18		
Mean (SD)	28.89 (22.146)	-14.57 (24.253)	37.43 (19.672)	-7.41 (25.565)		
Median	33.33	-11.11	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 44.4	0.0, 77.8	-77.8, 44.4		
Week 12						
n	52	52	25	24		
Mean (SD)	27.35 (20.341)	-15.81 (21.030)	47.11 (28.559)	-2.31 (31.932)		
Median	33.33	-11.11	44.44	0.00		
Min, Max	0.0, 66.7	-66.7, 22.2	0.0, 100.0	-77.8, 77.8		
Week 24						
n	49	49	24	23		
Mean (SD)	25.40 (23.570)	-17.46 (26.058)	35.65 (28.560)	-10.14 (30.871)		
Median	22.22	-22.22	33.33	-11.11		
Min, Max	0.0, 100.0	-77.8, 33.3	0.0, 100.0	-55.6, 77.8		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Fatigue

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	46	46	21	21	67	67
Mean (SD)	25.60 (23.292)	-16.18 (22.675)	42.33 (31.743)	-2.65 (29.795)	30.85 (27.135)	-11.94 (25.683)
Median	22.22	-11.11	33.33	0.00	33.33	-11.11
Min, Max	0.0, 100.0	-77.8, 33.3	0.0, 100.0	-55.6, 66.7	0.0, 100.0	-77.8, 66.7
Week 56						
n	34	34	17	16	51	50
Mean (SD)	30.39 (23.099)	-10.78 (26.448)	33.99 (14.418)	-11.81 (27.055)	31.59 (20.533)	-11.11 (26.370)
Median	33.33	-11.11	33.33	-11.11	33.33	-11.11
Min, Max	0.0, 100.0	-66.7, 44.4	11.1, 66.7	-66.7, 44.4	0.0, 100.0	-66.7, 44.4
Week 72						
n	21	21	12	11	33	32
Mean (SD)	25.93 (19.668)	-19.58 (21.054)	49.07 (22.453)	-1.01 (27.869)	34.34 (23.301)	-13.19 (24.835)
Median	22.22	-11.11	38.89	0.00	33.33	-11.11
Min, Max	0.0, 66.7	-66.7, 22.2	22.2, 100.0	-33.3, 66.7	0.0, 100.0	-66.7, 66.7

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Fatigue

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	20.63 (21.287)	-20.63 (25.359)	41.67 (22.810)	-13.89 (33.465)	28.28 (23.689)	-18.18 (27.972)
Median	11.11	-22.22	38.89	-16.67	33.33	-22.22
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 77.8	-66.7, 44.4	0.0, 77.8	-66.7, 44.4
Week 104						
n	4	4	2	2	6	6
Mean (SD)	13.89 (16.667)	-25.00 (24.637)	27.78 (7.857)	-38.89 (54.997)	18.52 (15.181)	-29.63 (31.946)
Median	11.11	-22.22	27.78	-38.89	22.22	-22.22
Min, Max	0.0, 33.3	-55.6, 0.0	22.2, 33.3	-77.8, 0.0	0.0, 33.3	-77.8, 0.0

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties. Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem. Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan. D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point. Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS
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Data cutoff date of 31Mar2023
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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Nausea and Vomiting

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	57		24		81	
Mean (SD)	2.63 (10.346)		6.25 (15.395)		3.70 (12.076)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	55	55	25	23		
Mean (SD)	3.94 (10.620)	1.21 (13.553)	7.33 (14.498)	0.00 (8.704)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 50.0	-50.0, 50.0	0.0, 50.0	-16.7, 16.7		
Week 4						
n	57	57	25	24		
Mean (SD)	4.09 (9.595)	1.46 (12.312)	3.33 (8.333)	-2.78 (16.051)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-50.0, 33.3	0.0, 33.3	-66.7, 16.7		

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Nausea and Vomiting

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	45	45	19	18		
Mean (SD)	4.81 (14.487)	1.48 (15.820)	6.14 (12.681)	0.93 (19.363)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 83.3	-33.3, 83.3	0.0, 50.0	-66.7, 33.3		
Week 12						
n	52	52	25	24		
Mean (SD)	3.21 (8.756)	0.64 (10.417)	8.00 (19.317)	0.00 (19.035)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-50.0, 33.3	0.0, 83.3	-66.7, 50.0		
Week 24						
n	49	49	24	23		
Mean (SD)	3.06 (11.115)	0.34 (13.815)	3.47 (6.914)	0.00 (12.309)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-50.0, 66.7	0.0, 16.7	-33.3, 16.7		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Nausea and Vomiting

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	46	46	21	21	67	67
Mean (SD)	4.71 (9.070)	3.26 (9.040)	4.76 (11.952)	1.59 (13.849)	4.73 (9.969)	2.74 (10.698)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-16.7, 33.3	0.0, 50.0	-16.7, 50.0	0.0, 50.0	-16.7, 50.0
Week 56						
n	34	34	17	16	51	50
Mean (SD)	0.49 (2.858)	-1.47 (7.530)	9.80 (24.338)	6.25 (23.472)	3.59 (14.649)	1.00 (14.835)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	-33.3, 16.7	0.0, 100.0	-33.3, 83.3	0.0, 100.0	-33.3, 83.3
Week 72						
n	21	21	12	11	33	32
Mean (SD)	0.79 (3.637)	-0.79 (6.404)	6.94 (11.143)	3.03 (17.979)	3.03 (7.744)	0.52 (11.581)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	-16.7, 16.7	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:48:23

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Nausea and Vomiting

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	1.19 (4.454)	0.00 (0.000)	8.33 (8.909)	2.08 (16.517)	3.79 (7.149)	0.76 (9.591)
Median	0.00	0.00	8.33	0.00	0.00	0.00
Min, Max	0.0, 16.7	0.0, 0.0	0.0, 16.7	-33.3, 16.7	0.0, 16.7	-33.3, 16.7
Week 104						
n	4	4	2	2	6	6
Mean (SD)	0.00 (0.000)	0.00 (0.000)	50.00 (23.570)	33.33 (0.000)	16.67 (27.889)	11.11 (17.213)
Median	0.00	0.00	50.00	33.33	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	33.3, 66.7	33.3, 33.3	0.0, 66.7	0.0, 33.3

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Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Pain

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	57		24		81	
Mean (SD)	14.62 (25.211)		20.83 (25.656)		16.46 (25.344)	
Median	0.00		16.67		0.00	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	55	55	24	22		
Mean (SD)	12.42 (23.399)	-2.73 (17.499)	24.31 (21.411)	1.52 (19.182)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 66.7	-33.3, 33.3		
Week 4						
n	57	57	25	24		
Mean (SD)	12.87 (21.826)	-1.75 (16.270)	20.67 (28.169)	0.69 (22.778)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 50.0	0.0, 100.0	-33.3, 83.3		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Pain

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	44	44	18	17		
Mean (SD)	12.50 (24.675)	1.52 (27.094)	10.19 (12.959)	-8.82 (17.793)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 33.3	-33.3, 16.7		
Week 12						
n	52	52	24	23		
Mean (SD)	11.22 (22.560)	-3.53 (20.435)	24.31 (29.480)	2.90 (30.423)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-83.3, 33.3	0.0, 100.0	-33.3, 83.3		
Week 24						
n	49	49	24	23		
Mean (SD)	11.56 (23.864)	-3.40 (23.318)	13.89 (21.234)	-5.80 (21.678)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-83.3, 66.7	0.0, 83.3	-66.7, 33.3		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:48:23

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Pain

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	46	46	21	21	67	67
Mean (SD)	13.04 (23.547)	-2.17 (20.064)	25.40 (34.001)	7.94 (32.753)	16.92 (27.599)	1.00 (24.938)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 83.3	-83.3, 33.3	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-83.3, 100.0
Week 56						
n	34	34	17	16	51	50
Mean (SD)	17.16 (26.744)	-1.96 (25.546)	25.49 (22.911)	6.25 (27.131)	19.93 (25.608)	0.67 (26.073)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 100.0	-83.3, 66.7	0.0, 66.7	-33.3, 66.7	0.0, 100.0	-83.3, 66.7
Week 72						
n	20	20	12	11	32	31
Mean (SD)	8.33 (16.667)	-9.17 (19.850)	22.22 (30.429)	9.09 (33.635)	13.54 (23.355)	-2.69 (26.561)
Median	0.00	0.00	8.33	0.00	0.00	0.00
Min, Max	0.0, 66.7	-50.0, 16.7	0.0, 100.0	-33.3, 83.3	0.0, 100.0	-50.0, 83.3

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Alexion Pharmaceuticals, Inc.
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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Pain

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	9.52 (19.298)	-8.33 (20.412)	25.00 (29.547)	16.67 (32.121)	15.15 (24.075)	0.76 (27.448)
Median	0.00	0.00	16.67	8.33	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 16.7	0.0, 83.3	-33.3, 66.7	0.0, 83.3	-66.7, 66.7
Week 104						
n	4	4	2	2	6	6
Mean (SD)	8.33 (16.667)	-16.67 (33.333)	16.67 (0.000)	8.33 (11.785)	11.11 (13.608)	-8.33 (29.345)
Median	0.00	0.00	16.67	8.33	8.33	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	16.7, 16.7	0.0, 16.7	0.0, 33.3	-66.7, 16.7

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Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023
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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Dyspnoea

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	57		24		81	
Mean (SD)	36.84 (28.650)		30.56 (29.352)		34.98 (28.820)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	56	56	25	23		
Mean (SD)	22.62 (26.291)	-14.88 (22.847)	33.33 (27.217)	4.35 (27.163)		
Median	33.33	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		
Week 4						
n	57	57	25	24		
Mean (SD)	14.62 (22.728)	-22.22 (25.459)	33.33 (33.333)	4.17 (33.060)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 66.7		

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Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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/alxn/a2040-pnh-301/a2040-pnh-301-gba/Files/tables/production/programs/t-eortc-summary.sas

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Dyspnoea

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	45	45	19	18		
Mean (SD)	18.52 (23.089)	-17.78 (24.205)	28.07 (27.807)	-5.56 (34.773)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 100.0	-100.0, 66.7		
Week 12						
n	52	52	25	24		
Mean (SD)	16.67 (21.390)	-19.87 (24.927)	29.33 (24.191)	-1.39 (39.902)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-100.0, 66.7		
Week 24						
n	49	49	24	23		
Mean (SD)	16.33 (22.692)	-19.05 (27.217)	15.28 (16.966)	-11.59 (25.837)		
Median	0.00	-33.33	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 33.3	-66.7, 33.3		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:48:23

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Dyspnoea

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	46	46	21	21	67	67
Mean (SD)	17.39 (24.077)	-19.57 (23.909)	15.87 (17.059)	-12.70 (22.301)	16.92 (21.999)	-17.41 (23.469)
Median	0.00	-33.33	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 100.0	-66.7, 33.3
Week 56						
n	34	34	17	16	51	50
Mean (SD)	22.55 (28.094)	-13.73 (26.102)	11.76 (16.420)	-8.33 (25.820)	18.95 (25.170)	-12.00 (25.873)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 100.0	-66.7, 33.3
Week 72						
n	21	21	12	11	33	32
Mean (SD)	14.29 (22.537)	-26.98 (20.053)	19.44 (26.432)	-9.09 (33.635)	16.16 (23.748)	-20.83 (26.437)
Median	0.00	-33.33	0.00	0.00	0.00	-33.33
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-66.7, 33.3

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Dyspnoea

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	7.14 (19.298)	-26.19 (23.310)	8.33 (15.430)	-16.67 (30.861)	7.58 (17.614)	-22.73 (25.996)
Median	0.00	-33.33	0.00	-16.67	0.00	-33.33
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-66.7, 33.3
Week 104						
n	4	4	2	2	6	6
Mean (SD)	0.00 (0.000)	-25.00 (31.914)	16.67 (23.570)	0.00 (0.000)	5.56 (13.608)	-16.67 (27.889)
Median	0.00	-16.67	16.67	0.00	0.00	0.00
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 33.3	0.0, 0.0	0.0, 33.3	-66.7, 0.0

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Run Date: 2024-04-26T15:48:23

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Insomnia

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	57		24		81	
Mean (SD)	26.32 (27.986)		29.17 (31.565)		27.16 (28.921)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	55	55	25	23		
Mean (SD)	21.82 (30.238)	-4.85 (19.687)	29.33 (32.375)	0.00 (31.782)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 66.7		
Week 4						
n	57	57	25	24		
Mean (SD)	21.05 (31.264)	-5.26 (24.226)	25.33 (30.852)	-4.17 (35.864)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-100.0, 66.7		

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Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Insomnia

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	45	45	19	18		
Mean (SD)	13.33 (19.332)	-12.59 (24.913)	15.79 (25.744)	-7.41 (31.427)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-100.0, 33.3		
Week 12						
n	52	52	25	24		
Mean (SD)	16.67 (24.254)	-9.62 (20.170)	30.67 (33.222)	1.39 (34.723)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 66.7		
Week 24						
n	49	49	24	23		
Mean (SD)	14.97 (25.514)	-10.20 (28.222)	20.83 (23.698)	-8.70 (25.060)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-100.0, 66.7	0.0, 66.7	-66.7, 33.3		

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Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Insomnia

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	46	46	21	21	67	67
Mean (SD)	20.29 (27.647)	-6.52 (23.954)	25.40 (33.174)	-4.76 (39.841)	21.89 (29.332)	-5.97 (29.545)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-100.0, 66.7	0.0, 100.0	-100.0, 66.7
Week 56						
n	34	34	17	16	51	50
Mean (SD)	21.57 (27.071)	-3.92 (25.643)	37.25 (33.087)	2.08 (33.264)	26.80 (29.829)	-2.00 (28.099)
Median	16.67	0.00	33.33	0.00	33.33	0.00
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7
Week 72						
n	21	21	12	11	33	32
Mean (SD)	12.70 (19.653)	-12.70 (19.653)	38.89 (34.329)	-3.03 (48.200)	22.22 (28.464)	-9.38 (31.943)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 100.0	-100.0, 66.7	0.0, 100.0	-100.0, 66.7

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Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Insomnia

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	9.52 (20.375)	-11.90 (16.575)	41.67 (42.725)	0.00 (47.140)	21.21 (33.405)	-7.58 (30.744)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7
Week 104						
n	4	4	2	2	6	6
Mean (SD)	8.33 (16.667)	-16.67 (33.333)	50.00 (70.711)	-16.67 (117.851)	22.22 (40.369)	-16.67 (58.689)
Median	0.00	0.00	50.00	-16.67	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 100.0	-100.0, 66.7	0.0, 100.0	-100.0, 66.7

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Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:48:23

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Post-hoc Analysen Studie ALPHA

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Appetite Loss

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	57		24		81	
Mean (SD)	8.77 (16.093)		18.06 (27.766)		11.52 (20.521)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 100.0		0.0, 100.0	
Week 2						
n	56	56	25	23		
Mean (SD)	10.12 (18.984)	1.19 (19.031)	20.00 (23.570)	1.45 (15.822)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-33.3, 33.3		
Week 4						
n	57	57	25	24		
Mean (SD)	5.85 (14.258)	-2.92 (14.476)	13.33 (27.217)	-4.17 (26.580)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-66.7, 66.7		

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Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Appetite Loss

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	45	45	19	18		
Mean (SD)	13.33 (19.332)	2.22 (20.597)	12.28 (19.909)	-3.70 (27.745)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-100.0, 33.3		
Week 12						
n	52	52	25	24		
Mean (SD)	7.05 (19.059)	-1.28 (24.665)	17.33 (29.059)	0.00 (24.077)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-66.7, 33.3		
Week 24						
n	49	49	24	23		
Mean (SD)	10.88 (22.961)	2.72 (28.736)	16.67 (24.077)	2.90 (13.901)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 100.0	0.0, 66.7	-33.3, 33.3		

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:48:23

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Appetite Loss

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	46	46	21	21	67	67
Mean (SD)	6.52 (13.370)	-0.72 (16.464)	19.05 (27.021)	6.35 (27.119)	10.45 (19.427)	1.49 (20.460)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0
Week 56						
n	34	34	17	16	51	50
Mean (SD)	7.84 (14.352)	1.96 (20.002)	11.76 (26.197)	-2.08 (25.730)	9.15 (18.948)	0.67 (21.811)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7
Week 72						
n	21	21	12	11	33	32
Mean (SD)	3.17 (10.026)	-4.76 (11.952)	19.44 (22.285)	3.03 (23.355)	9.09 (17.225)	-2.08 (16.801)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-33.3, 66.7

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Appetite Loss

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	7.14 (14.194)	0.00 (13.074)	16.67 (17.817)	0.00 (30.861)	10.61 (15.891)	0.00 (20.574)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3
Week 104						
n	4	4	2	2	6	6
Mean (SD)	0.00 (0.000)	0.00 (0.000)	66.67 (0.000)	16.67 (23.570)	22.22 (34.427)	5.56 (13.608)
Median	0.00	0.00	66.67	16.67	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	0.0, 33.3	0.0, 66.7	0.0, 33.3

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Constipation

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	57		24		81	
Mean (SD)	13.45 (23.452)		11.11 (18.822)		12.76 (22.091)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	55	55	25	23		
Mean (SD)	9.70 (22.837)	-4.24 (19.303)	17.33 (30.611)	5.80 (25.922)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		
Week 4						
n	57	57	25	24		
Mean (SD)	9.94 (22.682)	-3.51 (21.533)	18.67 (28.996)	8.33 (24.574)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-33.3, 66.7		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties. Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem. Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan. D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point. Baseline is defined as the last non-missing value prior to the first dose of study drug. Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023
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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Constipation

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	45	45	19	18		
Mean (SD)	5.93 (14.718)	-6.67 (20.841)	15.79 (30.161)	7.41 (14.260)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	0.0, 33.3		
Week 12						
n	52	52	25	24		
Mean (SD)	9.62 (24.106)	-3.85 (20.510)	17.33 (32.088)	6.94 (21.934)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-33.3, 66.7		
Week 24						
n	49	49	24	23		
Mean (SD)	6.80 (19.220)	-6.12 (20.039)	16.67 (26.006)	5.80 (21.678)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:48:23

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Constipation

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	46	46	21	21	67	67
Mean (SD)	6.52 (15.104)	-8.70 (21.585)	15.87 (22.655)	4.76 (21.822)	9.45 (18.171)	-4.48 (22.395)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-66.7, 66.7
Week 56						
n	34	34	17	16	51	50
Mean (SD)	9.80 (22.517)	-7.84 (20.199)	11.76 (20.211)	0.00 (12.172)	10.46 (21.592)	-5.33 (18.270)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-66.7, 33.3
Week 72						
n	21	21	12	11	33	32
Mean (SD)	12.70 (24.667)	-7.94 (20.829)	11.11 (16.412)	3.03 (17.979)	12.12 (21.760)	-4.17 (20.302)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 33.3

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Constipation

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	14.29 (17.118)	-4.76 (22.100)	25.00 (46.291)	16.67 (43.644)	18.18 (30.389)	3.03 (32.382)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0
Week 104						
n	4	4	2	2	6	6
Mean (SD)	16.67 (19.245)	-8.33 (16.667)	33.33 (47.140)	0.00 (47.140)	22.22 (27.217)	-5.56 (25.092)
Median	16.67	0.00	33.33	0.00	16.67	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Diarrhea

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	57		24		81	
Mean (SD)	5.26 (16.414)		12.50 (21.563)		7.41 (18.257)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	56	56	25	23		
Mean (SD)	5.95 (18.099)	0.60 (17.397)	5.33 (15.753)	-7.25 (22.375)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 100.0	0.0, 66.7	-66.7, 33.3		
Week 4						
n	57	57	25	24		
Mean (SD)	5.26 (13.786)	0.00 (19.920)	4.00 (11.055)	-8.33 (20.264)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-100.0, 66.7	0.0, 33.3	-66.7, 33.3		

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Diarrhea

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	45	45	19	18		
Mean (SD)	7.41 (15.713)	2.22 (19.332)	7.02 (17.843)	-7.41 (18.277)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 66.7	0.0, 66.7	-33.3, 33.3		
Week 12						
n	52	52	25	24		
Mean (SD)	5.77 (12.733)	0.00 (16.169)	4.00 (14.657)	-8.33 (28.233)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-66.7, 66.7		
Week 24						
n	49	49	24	23		
Mean (SD)	5.44 (12.448)	0.68 (15.942)	4.17 (11.261)	-7.25 (17.281)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 0.0		

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Diarrhea

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	46	46	21	21	67	67
Mean (SD)	5.07 (12.105)	-1.45 (21.031)	7.94 (14.548)	-4.76 (24.234)	5.97 (12.878)	-2.49 (21.953)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-100.0, 33.3
Week 56						
n	34	34	17	16	51	50
Mean (SD)	6.86 (13.681)	1.96 (21.620)	7.84 (14.575)	-10.42 (23.472)	7.19 (13.846)	-2.00 (22.748)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-100.0, 33.3
Week 72						
n	20	20	12	11	32	31
Mean (SD)	0.00 (0.000)	-1.67 (7.454)	25.00 (32.177)	6.06 (35.957)	9.38 (22.773)	1.08 (21.915)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:48:23

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Diarrhea

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	2.38 (8.909)	2.38 (8.909)	8.33 (15.430)	-8.33 (29.547)	4.55 (11.708)	-1.52 (19.182)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3
Week 104						
n	4	4	2	2	6	6
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0

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Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:48:23

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Financial Difficulties

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	57		24		81	
Mean (SD)	20.47 (30.702)		23.61 (31.819)		21.40 (30.871)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	57	57	25	23		
Mean (SD)	18.13 (29.594)	-2.34 (15.249)	24.00 (34.048)	2.90 (28.272)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 100.0		
Week 4						
n	57	57	25	24		
Mean (SD)	17.54 (26.804)	-2.92 (20.198)	18.67 (33.444)	-4.17 (29.996)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 100.0		

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Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Financial Difficulties

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	45	45	19	18		
Mean (SD)	12.59 (21.660)	-6.67 (22.019)	14.04 (23.083)	0.00 (19.803)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 66.7	-33.3, 33.3		
Week 12						
n	52	52	25	24		
Mean (SD)	17.95 (26.782)	-2.56 (17.272)	22.67 (35.642)	0.00 (32.601)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 100.0		
Week 24						
n	49	49	24	23		
Mean (SD)	17.69 (28.949)	-2.72 (25.310)	22.22 (34.983)	-1.45 (29.264)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-100.0, 100.0	0.0, 100.0	-66.7, 100.0		

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Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Financial Difficulties

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	46	46	21	21	67	67
Mean (SD)	17.39 (26.979)	-5.07 (23.272)	20.63 (32.449)	-6.35 (35.931)	18.41 (28.594)	-5.47 (27.583)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-100.0, 100.0	0.0, 100.0	-100.0, 100.0
Week 56						
n	34	34	17	16	51	50
Mean (SD)	12.75 (23.232)	-8.82 (18.908)	25.49 (34.419)	-2.08 (39.382)	16.99 (27.787)	-6.67 (26.937)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 100.0	0.0, 100.0	-66.7, 100.0
Week 72						
n	20	20	12	11	32	31
Mean (SD)	8.33 (14.809)	-10.00 (24.423)	11.11 (21.711)	-15.15 (22.918)	9.38 (17.422)	-11.83 (23.646)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-66.7, 0.0	0.0, 66.7	-66.7, 33.3

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Source: ADaM.ADSL, ADaM.ADQS

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Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Financial Difficulties

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	9.52 (15.627)	-9.52 (20.375)	8.33 (15.430)	-25.00 (29.547)	9.09 (15.195)	-15.15 (24.618)
Median	0.00	0.00	0.00	-16.67	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0
Week 104						
n	4	4	2	2	6	6
Mean (SD)	0.00 (0.000)	-16.67 (33.333)	16.67 (23.570)	-66.67 (0.000)	5.56 (13.608)	-33.33 (36.515)
Median	0.00	0.00	16.67	-66.67	0.00	-33.33
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 33.3	-66.7, -66.7	0.0, 33.3	-66.7, 0.0

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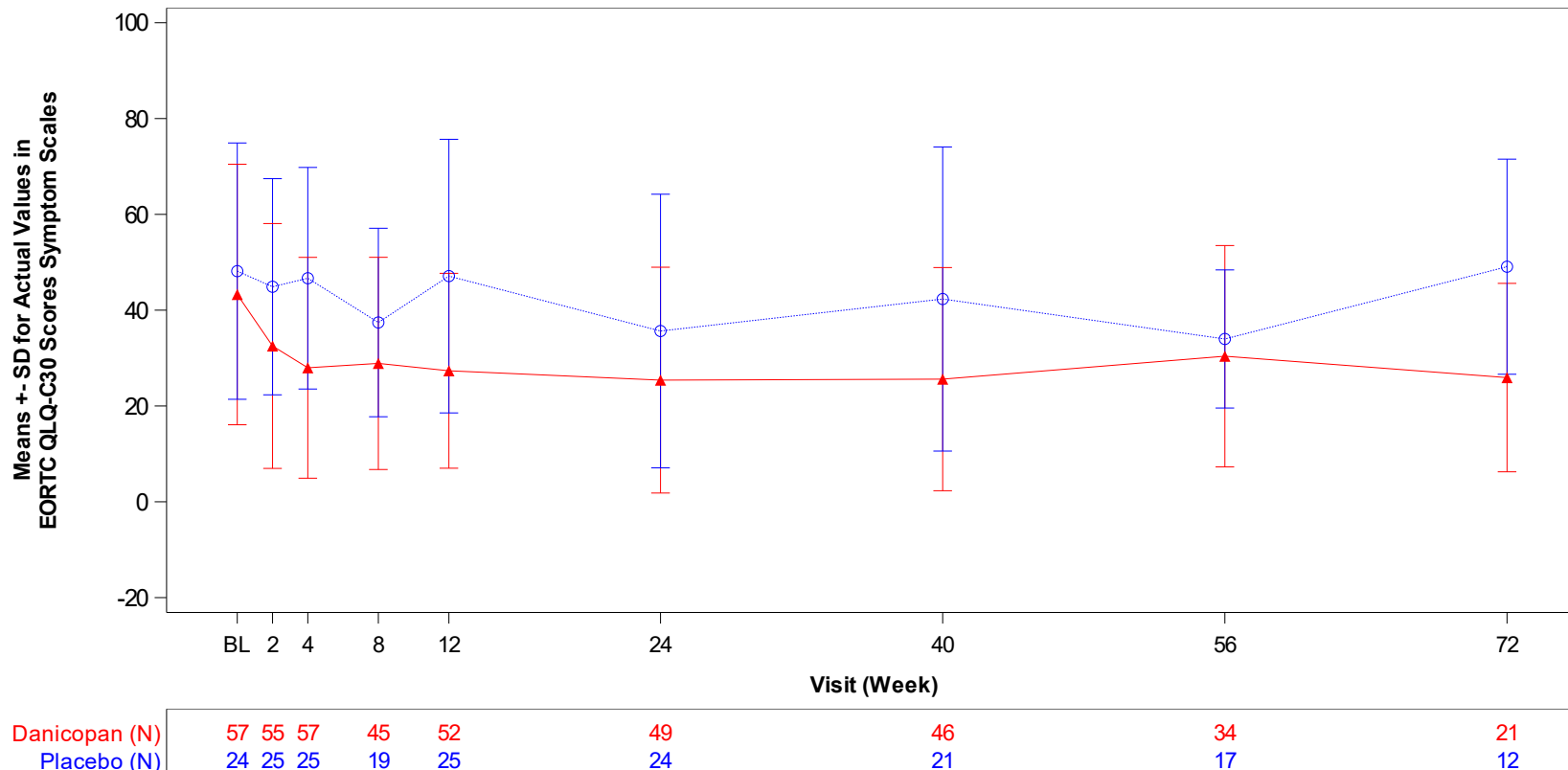
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Figure GERMANY.2.2.6.3
Plots of Mean +- SD for Actual Values in EORTC QLQ-C30 Scores Symptom Scales During 72 Weeks
Modified Randomized Set

Fatigue



Baseline is defined as the last non-missing value prior to the first dose of study drug.

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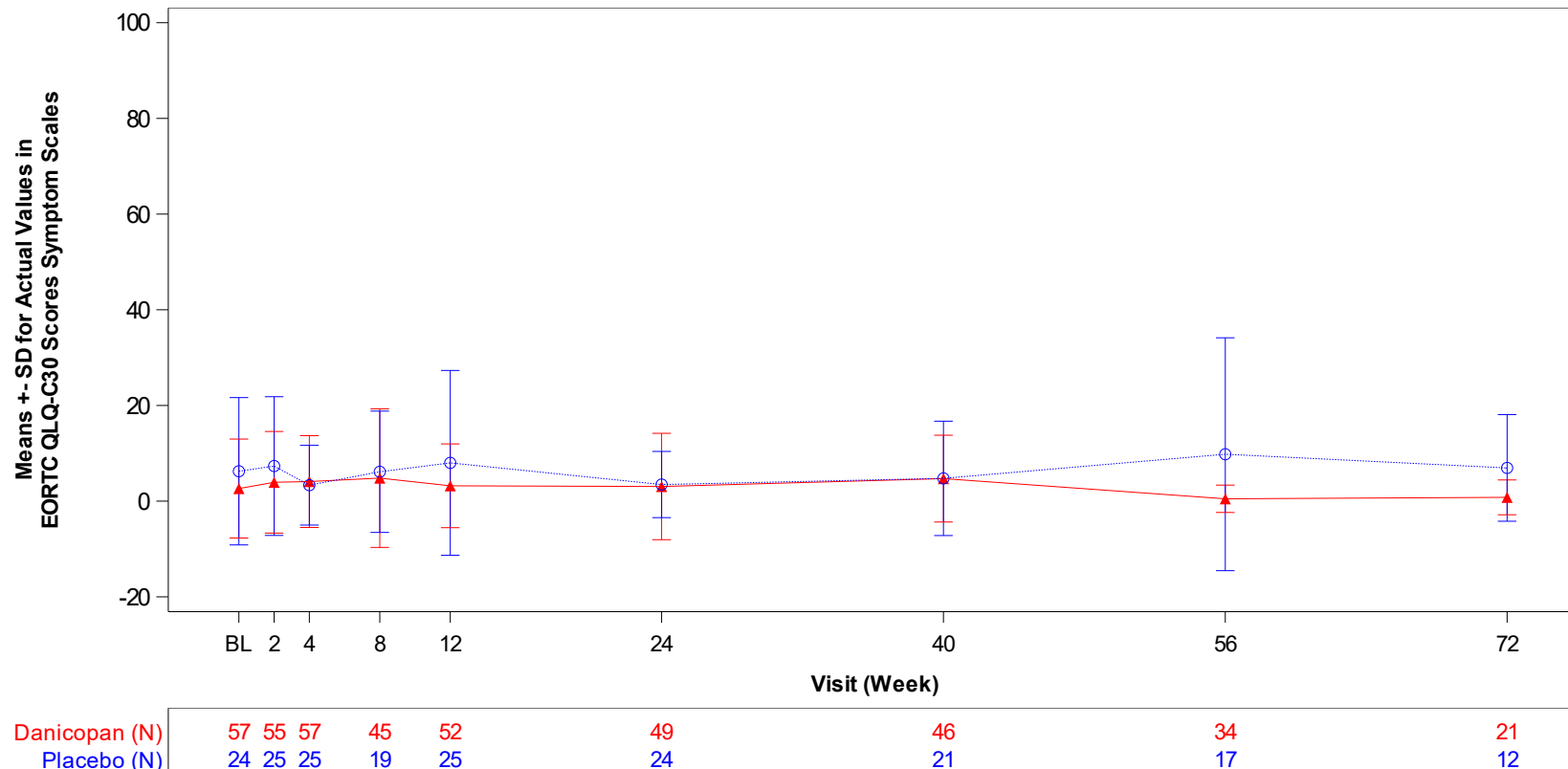
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Figure GERMANY.2.2.6.3
Plots of Mean +- SD for Actual Values in EORTC QLQ-C30 Scores Symptom Scales During 72 Weeks
Modified Randomized Set

Nausea and Vomiting



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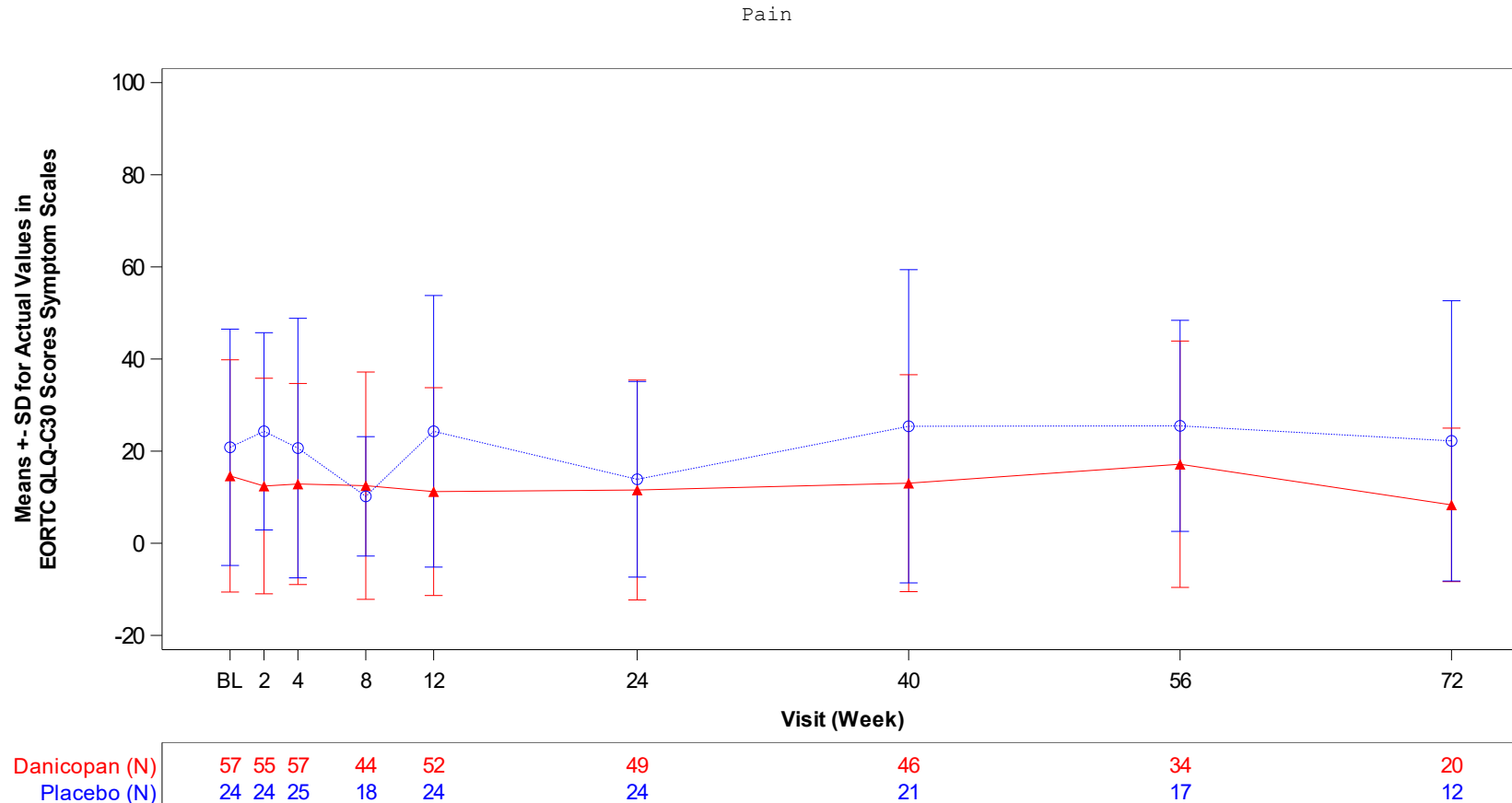
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Figure GERMANY.2.2.6.3
Plots of Mean +- SD for Actual Values in EORTC QLQ-C30 Scores Symptom Scales During 72 Weeks
Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.
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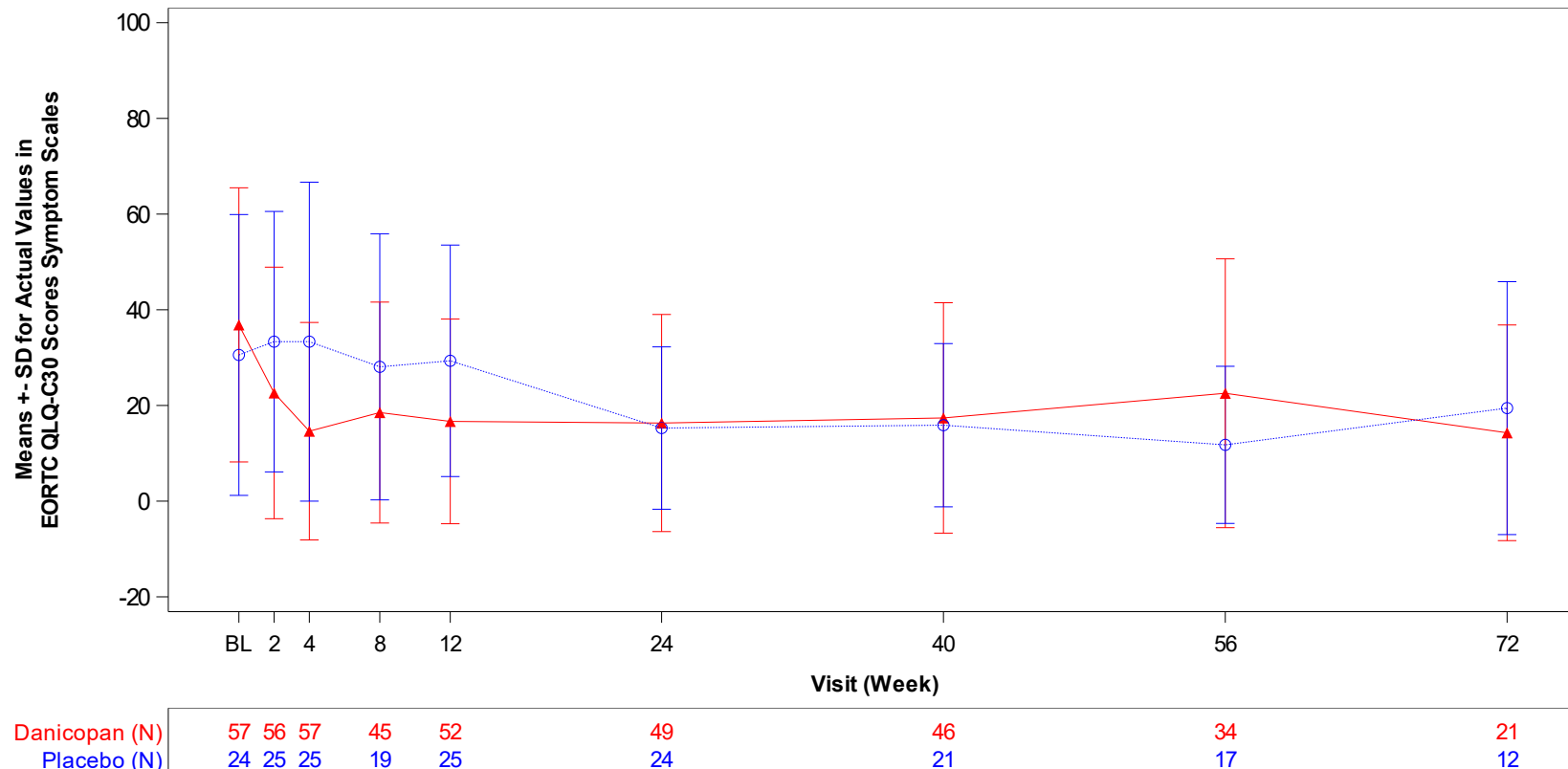
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Figure GERMANY.2.2.6.3
Plots of Mean \pm SD for Actual Values in EORTC QLQ-C30 Scores Symptom Scales During 72 Weeks
Modified Randomized Set

Dyspnoea



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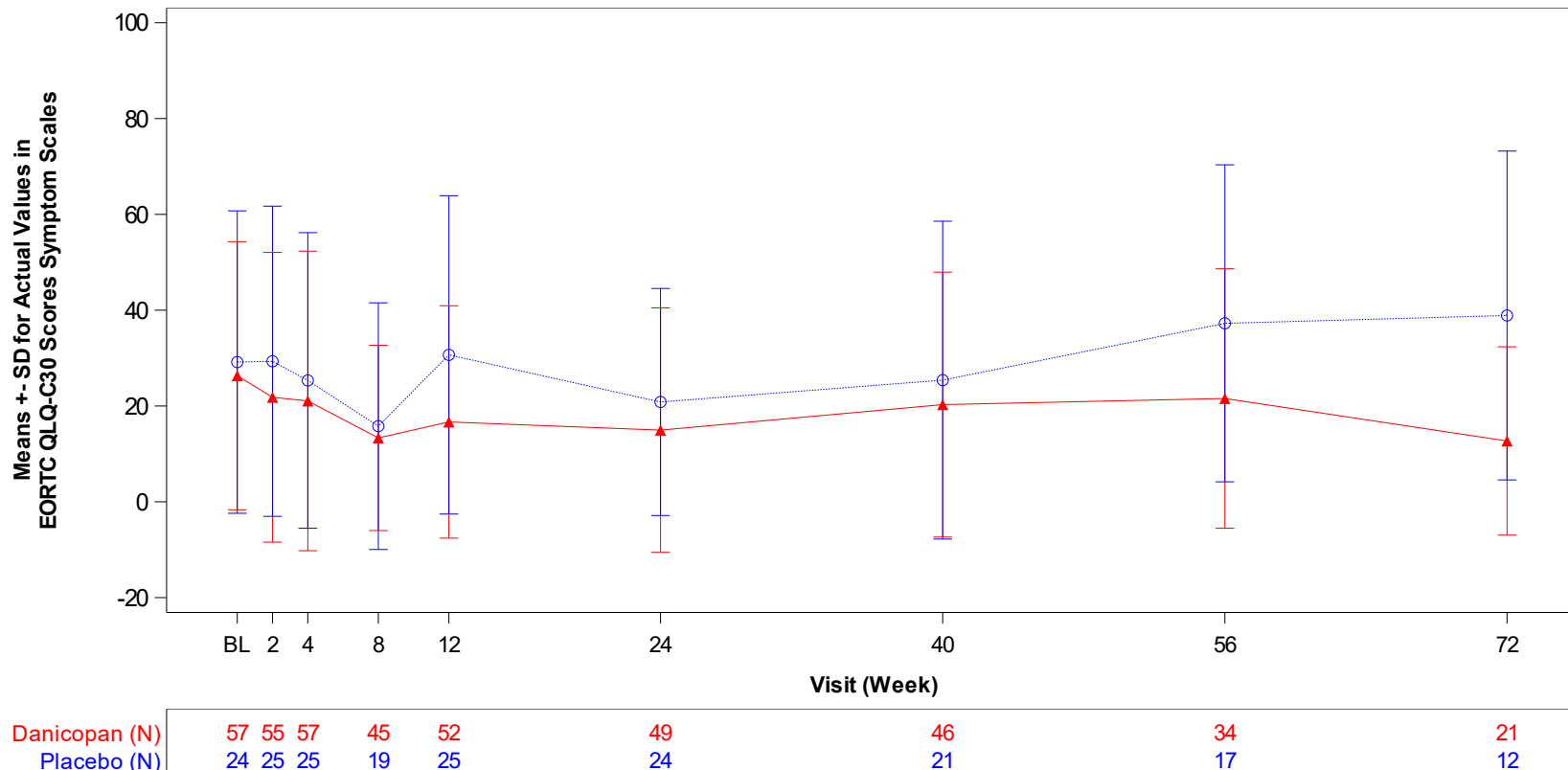
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Figure GERMANY.2.2.6.3
Plots of Mean +- SD for Actual Values in EORTC QLQ-C30 Scores Symptom Scales During 72 Weeks
Modified Randomized Set

Insomnia



Baseline is defined as the last non-missing value prior to the first dose of study drug.
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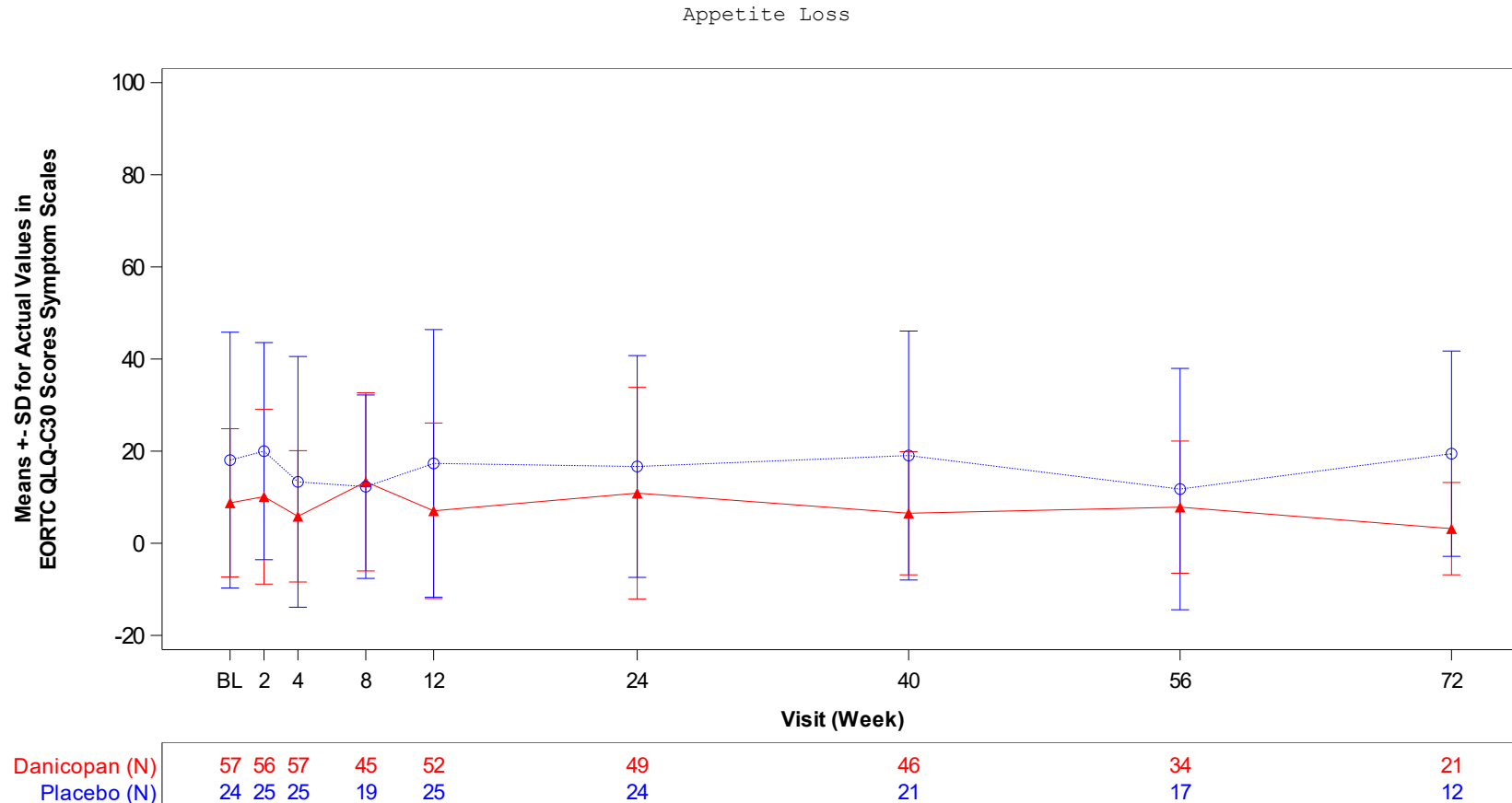
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Figure GERMANY.2.2.6.3
Plots of Mean +- SD for Actual Values in EORTC QLQ-C30 Scores Symptom Scales During 72 Weeks
Modified Randomized Set



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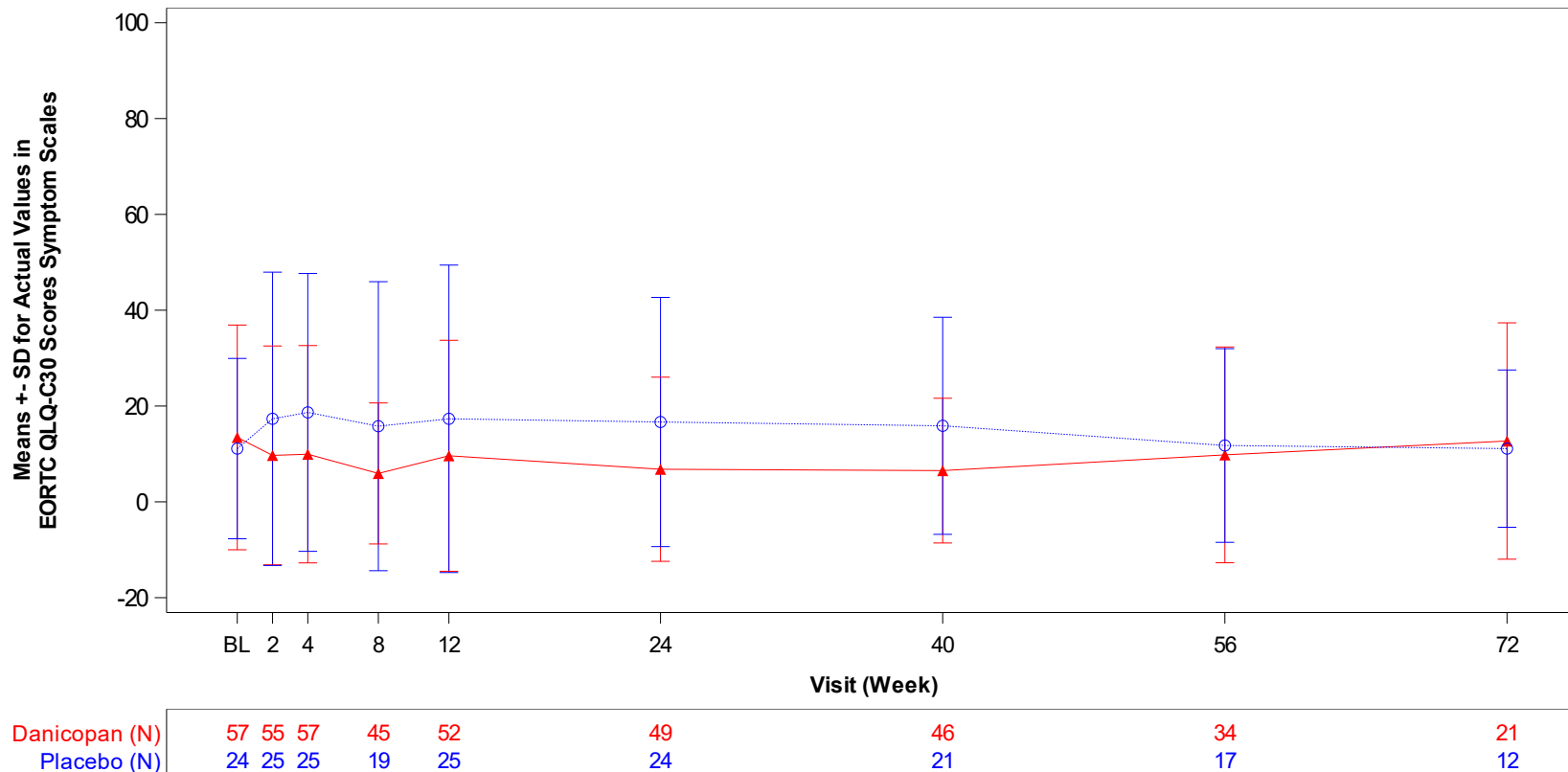
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Figure GERMANY.2.2.6.3
Plots of Mean +- SD for Actual Values in EORTC QLQ-C30 Scores Symptom Scales During 72 Weeks
Modified Randomized Set

Constipation



Baseline is defined as the last non-missing value prior to the first dose of study drug.
EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.
Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Source: ADaM.ADQS

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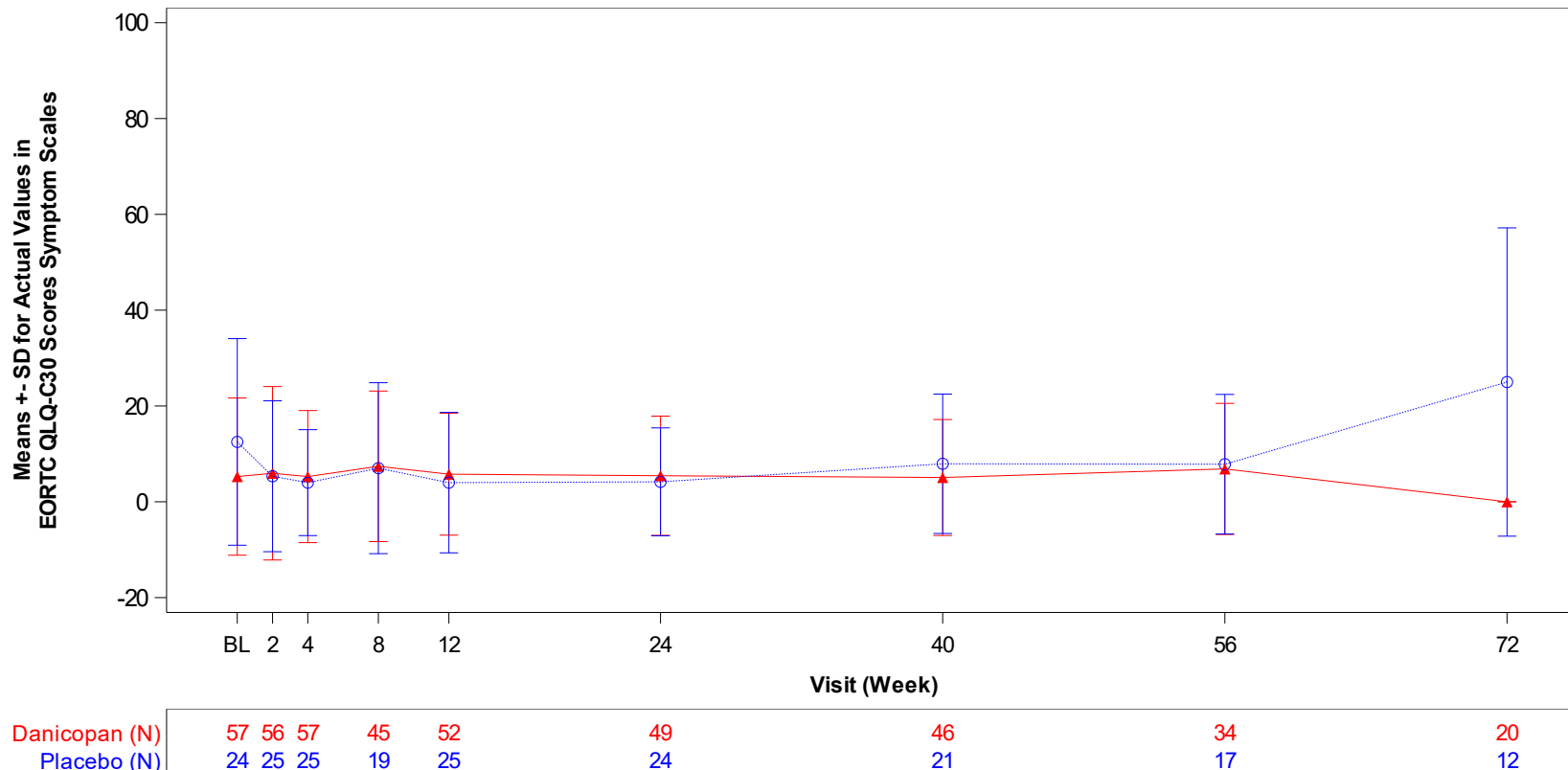
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Figure GERMANY.2.2.6.3
Plots of Mean +- SD for Actual Values in EORTC QLQ-C30 Scores Symptom Scales During 72 Weeks
Modified Randomized Set

Diarrhea



Baseline is defined as the last non-missing value prior to the first dose of study drug.
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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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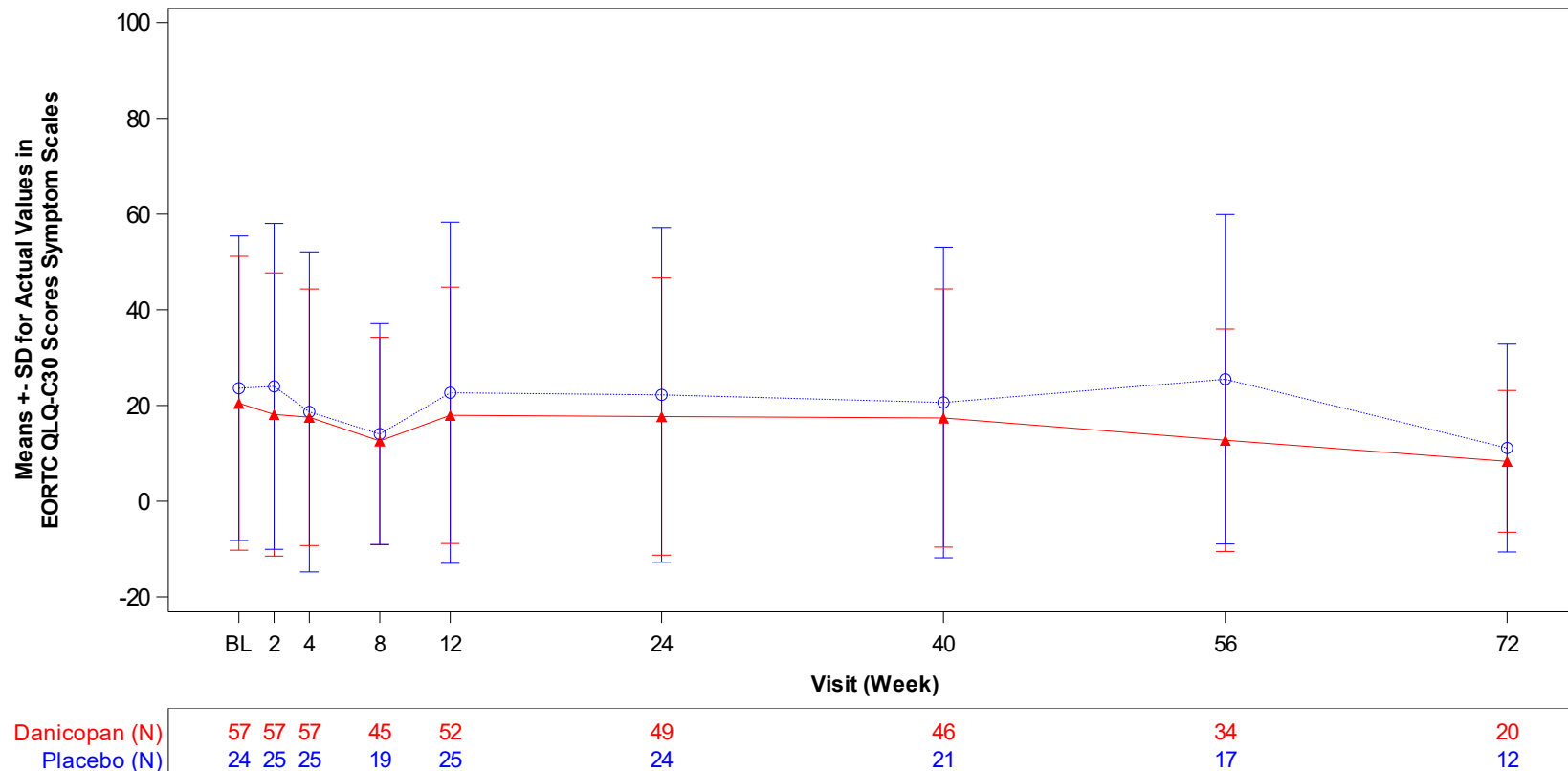
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Figure GERMANY.2.2.6.3
Plots of Mean +- SD for Actual Values in EORTC QLQ-C30 Scores Symptom Scales During 72 Weeks
Modified Randomized Set

Financial Difficulties



Baseline is defined as the last non-missing value prior to the first dose of study drug.
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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Table GERMANY.2.5.2.1
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-11.79 (2.636)	0.42 (4.060)	-12.21 (4.755)	0.0123
	95% CI for LS MEAN	(-17.04, -6.54)	(-7.67, 8.51)	(-21.69, -2.73)	
Week 4	LS Mean (SEM)	-16.28 (2.623)	1.49 (4.016)	-17.77 (4.712)	0.0003
	95% CI for LS MEAN	(-21.50, -11.06)	(-6.50, 9.49)	(-27.15, -8.39)	
Week 8	LS Mean (SEM)	-15.51 (2.864)	-2.24 (4.448)	-13.27 (5.196)	0.0131
	95% CI for LS MEAN	(-21.24, -9.79)	(-11.12, 6.64)	(-23.66, -2.89)	
Week 12	LS Mean (SEM)	-15.68 (2.798)	-0.82 (4.200)	-14.86 (4.965)	0.0038
	95% CI for LS MEAN	(-21.25, -10.11)	(-9.19, 7.54)	(-24.75, -4.96)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). An Unstructured covariance structure was used.

P-value is from difference of LSM from MMRM model.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.1
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	0.76 (1.438)	2.18 (2.220)	-1.42 (2.611)	0.5872
	95% CI for LS MEAN	(-2.11, 3.62)	(-2.24, 6.60)	(-6.62, 3.78)	
Week 4	LS Mean (SEM)	0.92 (1.270)	-0.74 (1.939)	1.66 (2.281)	0.4690
	95% CI for LS MEAN	(-1.61, 3.45)	(-4.61, 3.12)	(-2.88, 6.20)	
Week 8	LS Mean (SEM)	1.56 (2.133)	3.97 (3.313)	-2.41 (3.912)	0.5411
	95% CI for LS MEAN	(-2.71, 5.83)	(-2.66, 10.60)	(-10.24, 5.43)	
Week 12	LS Mean (SEM)	1.42 (1.790)	2.03 (2.676)	-0.62 (3.192)	0.8477
	95% CI for LS MEAN	(-2.16, 5.00)	(-3.32, 7.39)	(-7.01, 5.78)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). An Unstructured covariance structure was used.

P-value is from difference of LSM from MMRM model.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.1
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.33 (2.134)	4.29 (3.358)	-6.61 (3.913)	0.0951
	95% CI for LS MEAN	(-6.58, 1.92)	(-2.40, 10.97)	(-14.41, 1.18)	
Week 4	LS Mean (SEM)	-1.71 (2.262)	3.01 (3.465)	-4.72 (4.072)	0.2500
	95% CI for LS MEAN	(-6.21, 2.80)	(-3.89, 9.91)	(-12.83, 3.39)	
Week 8	LS Mean (SEM)	0.18 (3.504)	-3.81 (5.597)	3.99 (6.557)	0.5452
	95% CI for LS MEAN	(-6.84, 7.20)	(-15.03, 7.40)	(-9.15, 17.14)	
Week 12	LS Mean (SEM)	-2.41 (2.876)	5.80 (4.375)	-8.21 (5.185)	0.1177
	95% CI for LS MEAN	(-8.14, 3.32)	(-2.92, 14.52)	(-18.54, 2.13)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). An Unstructured covariance structure was used.

P-value is from difference of LSM from MMRM model.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.1
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-14.06 (2.942)	1.19 (4.569)	-15.25 (5.327)	0.0055
	95% CI for LS MEAN	(-19.92, -8.20)	(-7.91, 10.29)	(-25.87, -4.63)	
Week 4	LS Mean (SEM)	-21.79 (3.126)	0.86 (4.803)	-22.64 (5.634)	0.0001
	95% CI for LS MEAN	(-28.01, -15.57)	(-8.70, 10.41)	(-33.86, -11.43)	
Week 8	LS Mean (SEM)	-18.22 (3.193)	-5.25 (4.971)	-12.96 (5.806)	0.0289
	95% CI for LS MEAN	(-24.59, -11.85)	(-15.17, 4.66)	(-24.55, -1.37)	
Week 12	LS Mean (SEM)	-18.78 (3.098)	-4.70 (4.608)	-14.08 (5.452)	0.0119
	95% CI for LS MEAN	(-24.95, -12.60)	(-13.89, 4.49)	(-24.95, -3.20)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). An Unstructured covariance structure was used.

P-value is from difference of LSM from MMRM model.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.1
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-3.99 (3.176)	0.96 (4.892)	-4.95 (5.729)	0.3908
	95% CI for LS MEAN	(-10.32, 2.35)	(-8.79, 10.71)	(-16.37, 6.48)	
Week 4	LS Mean (SEM)	-4.83 (3.491)	-2.29 (5.351)	-2.54 (6.296)	0.6873
	95% CI for LS MEAN	(-11.78, 2.11)	(-12.94, 8.36)	(-15.08, 9.99)	
Week 8	LS Mean (SEM)	-11.44 (3.206)	-4.86 (5.001)	-6.57 (5.819)	0.2633
	95% CI for LS MEAN	(-17.85, -5.02)	(-14.87, 5.14)	(-18.23, 5.08)	
Week 12	LS Mean (SEM)	-8.61 (3.150)	3.27 (4.676)	-11.88 (5.533)	0.0351
	95% CI for LS MEAN	(-14.88, -2.34)	(-6.05, 12.58)	(-22.90, -0.85)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). An Unstructured covariance structure was used.

P-value is from difference of LSM from MMRM model.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.1
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.48 (2.241)	3.88 (3.481)	-4.36 (4.086)	0.2894
	95% CI for LS MEAN	(-4.94, 3.98)	(-3.05, 10.81)	(-12.49, 3.77)	
Week 4	LS Mean (SEM)	-4.71 (2.215)	-1.86 (3.395)	-2.85 (4.002)	0.4787
	95% CI for LS MEAN	(-9.12, -0.30)	(-8.62, 4.89)	(-10.82, 5.12)	
Week 8	LS Mean (SEM)	2.18 (2.969)	2.25 (4.626)	-0.07 (5.454)	0.9905
	95% CI for LS MEAN	(-3.75, 8.12)	(-6.99, 11.49)	(-10.97, 10.84)	
Week 12	LS Mean (SEM)	-1.94 (3.050)	2.30 (4.586)	-4.24 (5.469)	0.4408
	95% CI for LS MEAN	(-8.01, 4.14)	(-6.84, 11.44)	(-15.14, 6.66)	

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Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). An Unstructured covariance structure was used.

P-value is from difference of LSM from MMRM model.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.1
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.30 (2.745)	6.70 (4.213)	-9.00 (4.936)	0.0722
	95% CI for LS MEAN	(-7.76, 3.17)	(-1.68, 15.09)	(-18.83, 0.83)	
Week 4	LS Mean (SEM)	-1.96 (2.813)	9.42 (4.303)	-11.38 (5.052)	0.0271
	95% CI for LS MEAN	(-7.55, 3.64)	(0.86, 17.99)	(-21.44, -1.32)	
Week 8	LS Mean (SEM)	-5.67 (2.604)	8.35 (4.065)	-14.02 (4.710)	0.0042
	95% CI for LS MEAN	(-10.88, -0.46)	(0.23, 16.47)	(-23.44, -4.60)	
Week 12	LS Mean (SEM)	-1.93 (2.785)	8.03 (4.158)	-9.96 (4.913)	0.0462
	95% CI for LS MEAN	(-7.47, 3.62)	(-0.25, 16.32)	(-19.75, -0.17)	

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Table GERMANY.2.5.2.1
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.90 (2.200)	-2.78 (3.422)	1.88 (4.040)	0.6426
	95% CI for LS MEAN	(-5.28, 3.48)	(-9.60, 4.03)	(-6.16, 9.93)	
Week 4	LS Mean (SEM)	-1.53 (1.771)	-4.28 (2.718)	2.76 (3.210)	0.3929
	95% CI for LS MEAN	(-5.05, 2.00)	(-9.69, 1.13)	(-3.63, 9.15)	
Week 8	LS Mean (SEM)	1.00 (2.243)	-1.91 (3.533)	2.91 (4.152)	0.4854
	95% CI for LS MEAN	(-3.47, 5.48)	(-8.96, 5.14)	(-5.38, 11.20)	
Week 12	LS Mean (SEM)	-0.94 (1.895)	-4.28 (2.785)	3.34 (3.334)	0.3201
	95% CI for LS MEAN	(-4.72, 2.83)	(-9.84, 1.27)	(-3.31, 9.99)	

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Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). An Unstructured covariance structure was used.

P-value is from difference of LSM from MMRM model.

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Table GERMANY.2.5.2.1
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.79 (2.607)	3.49 (4.042)	-5.29 (4.705)	0.2650
	95% CI for LS MEAN	(-6.99, 3.40)	(-4.56, 11.54)	(-14.66, 4.09)	
Week 4	LS Mean (SEM)	-2.38 (2.817)	-2.59 (4.318)	0.21 (5.064)	0.9672
	95% CI for LS MEAN	(-7.98, 3.23)	(-11.18, 6.01)	(-9.87, 10.29)	
Week 8	LS Mean (SEM)	-6.12 (2.791)	0.63 (4.347)	-6.75 (5.058)	0.1882
	95% CI for LS MEAN	(-11.72, -0.52)	(-8.09, 9.35)	(-16.91, 3.41)	
Week 12	LS Mean (SEM)	-2.15 (2.855)	1.58 (4.313)	-3.73 (5.081)	0.4646
	95% CI for LS MEAN	(-7.83, 3.53)	(-7.00, 10.17)	(-13.85, 6.38)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). An Unstructured covariance structure was used.

P-value is from difference of LSM from MMRM model.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Data cutoff date of 31Mar2023

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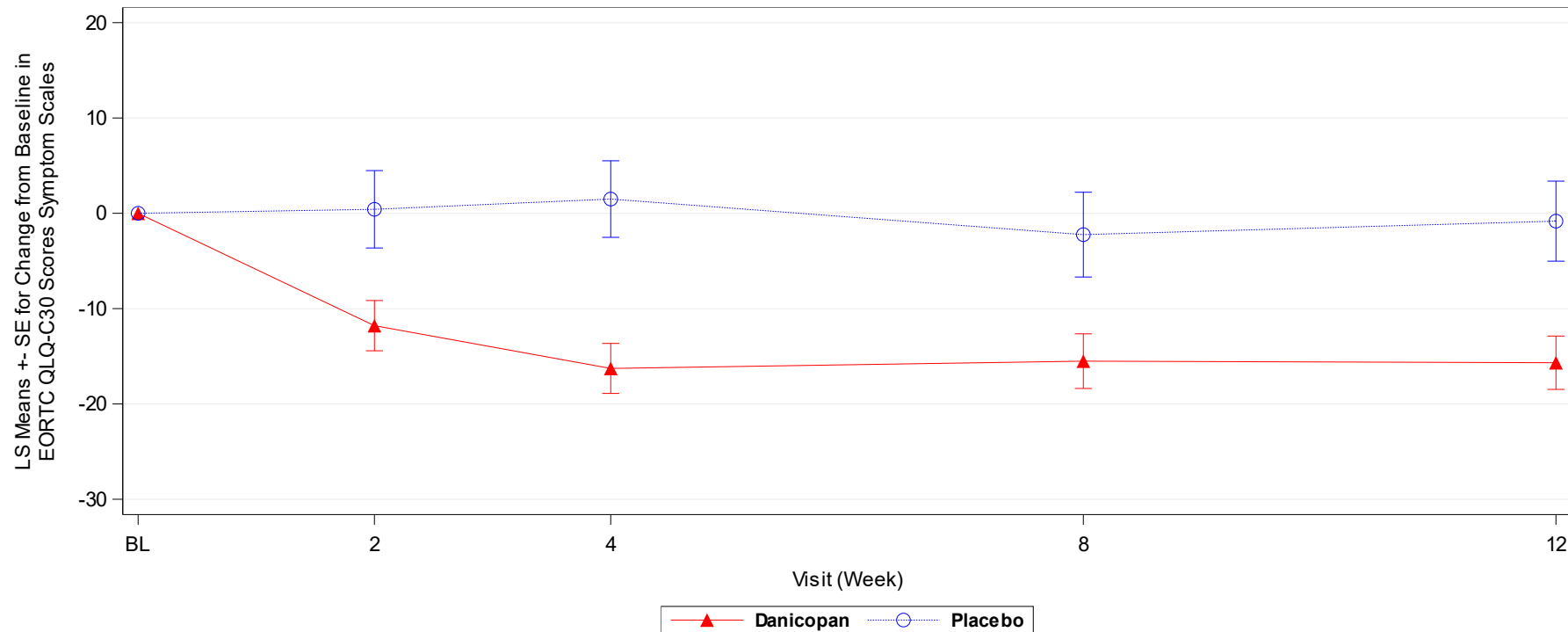
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Figure GERMANY.2.2.6.1
Plots of LS Means \pm SE for Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During 12-Week of Treatment Period 1
Modified Randomized Set

Fatigue



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Source: ADaM.ADSL, ADaM.ADQS

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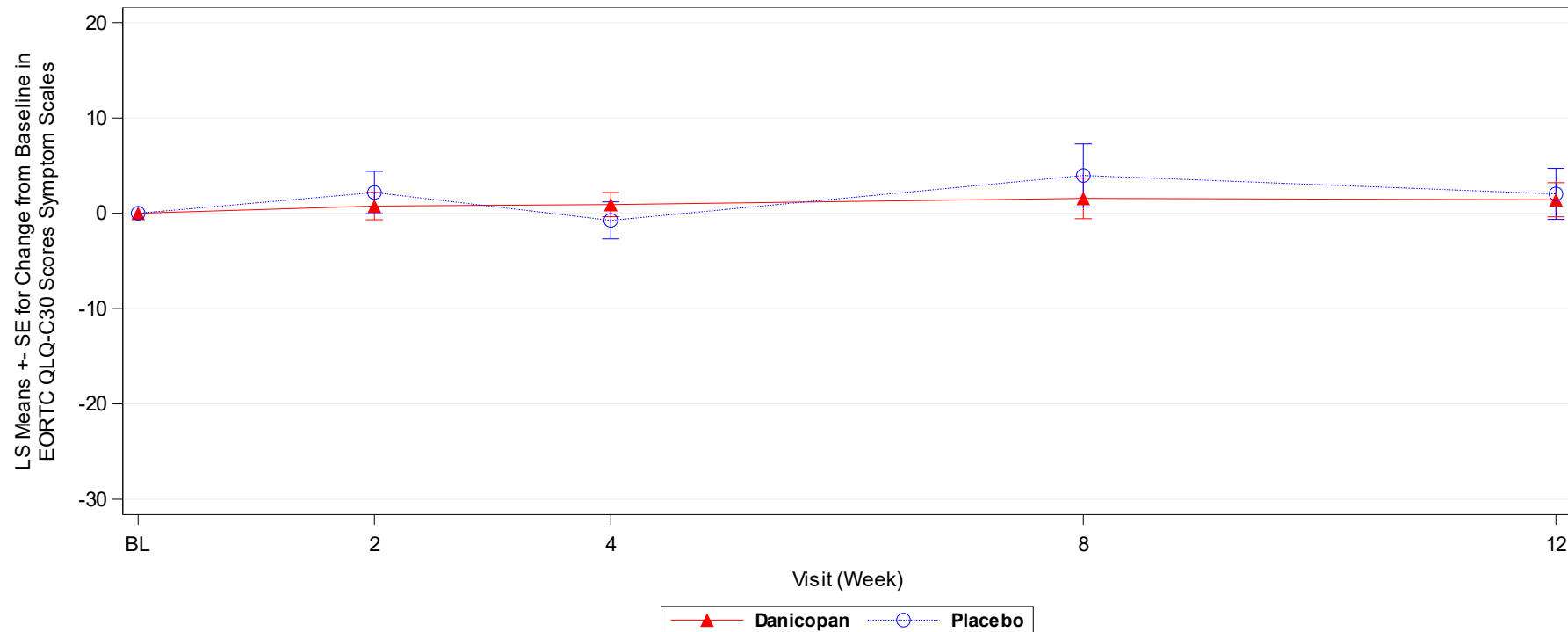
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Figure GERMANY.2.2.6.1
Plots of LS Means +- SE for Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During 12-Week of Treatment Period 1
Modified Randomized Set

Nausea and Vomiting



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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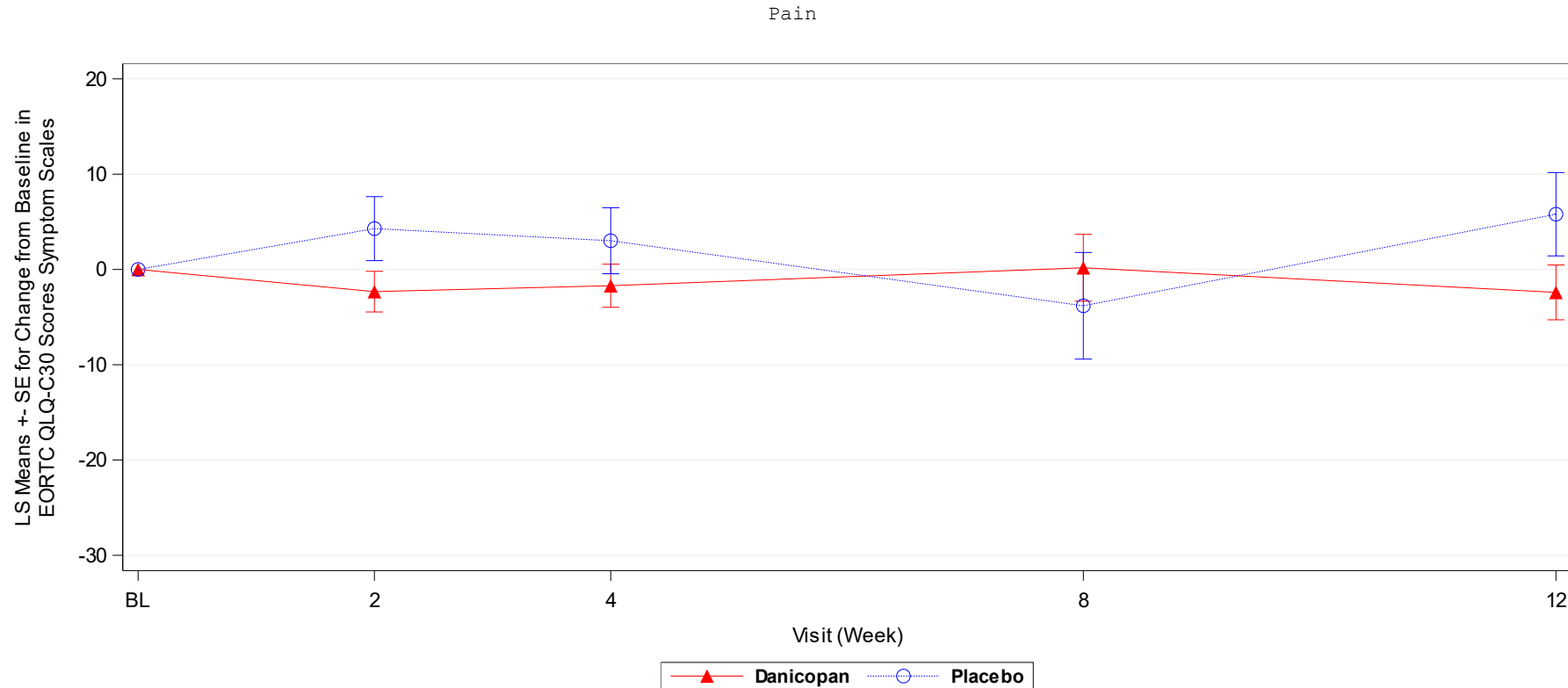
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Figure GERMANY.2.2.6.1
Plots of LS Means +- SE for Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During 12-Week of Treatment Period 1
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Source: ADaM.ADSL, ADaM.ADQS

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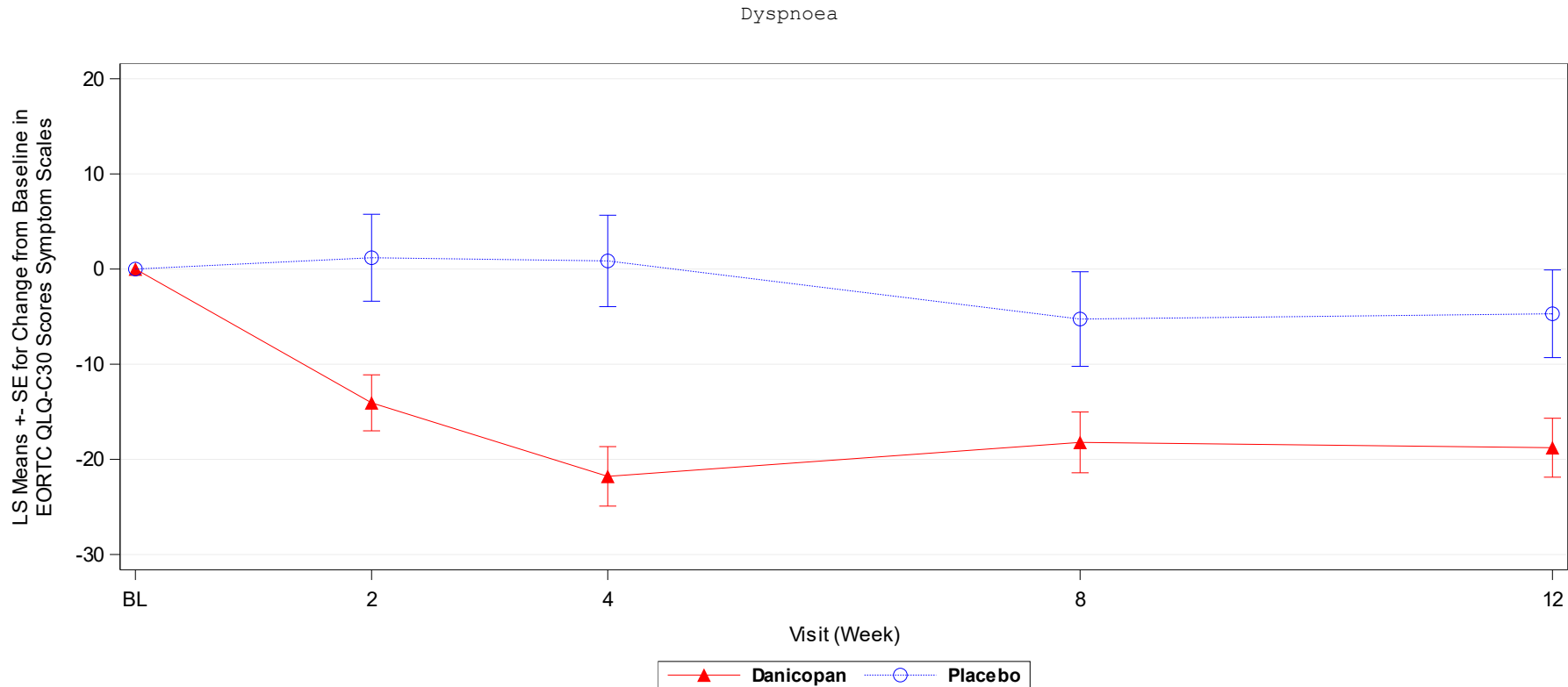
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Figure GERMANY.2.2.6.1
Plots of LS Means \pm SE for Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During 12-Week of Treatment Period 1
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Source: ADaM.ADSL, ADaM.ADQS

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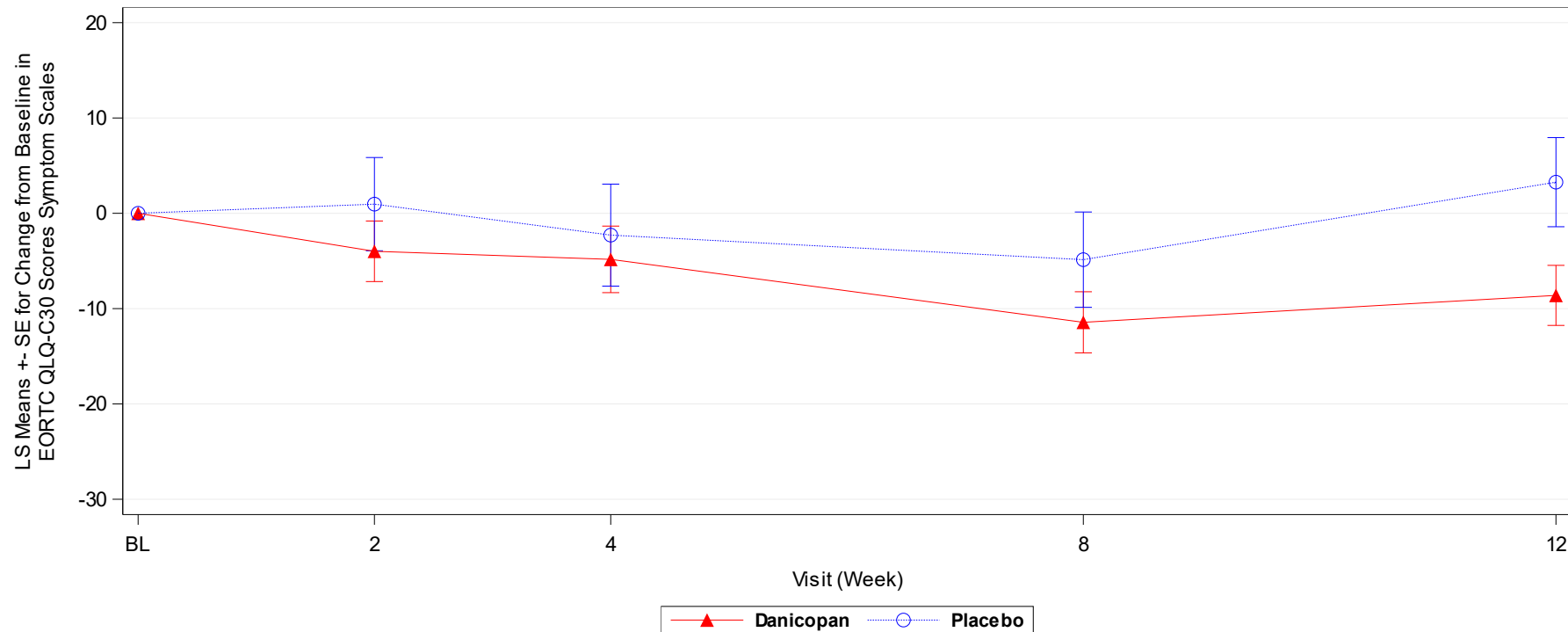
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Figure GERMANY.2.2.6.1
Plots of LS Means \pm SE for Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During 12-Week of Treatment Period 1
Modified Randomized Set

Insomnia



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Source: ADaM.ADSL, ADaM.ADQS

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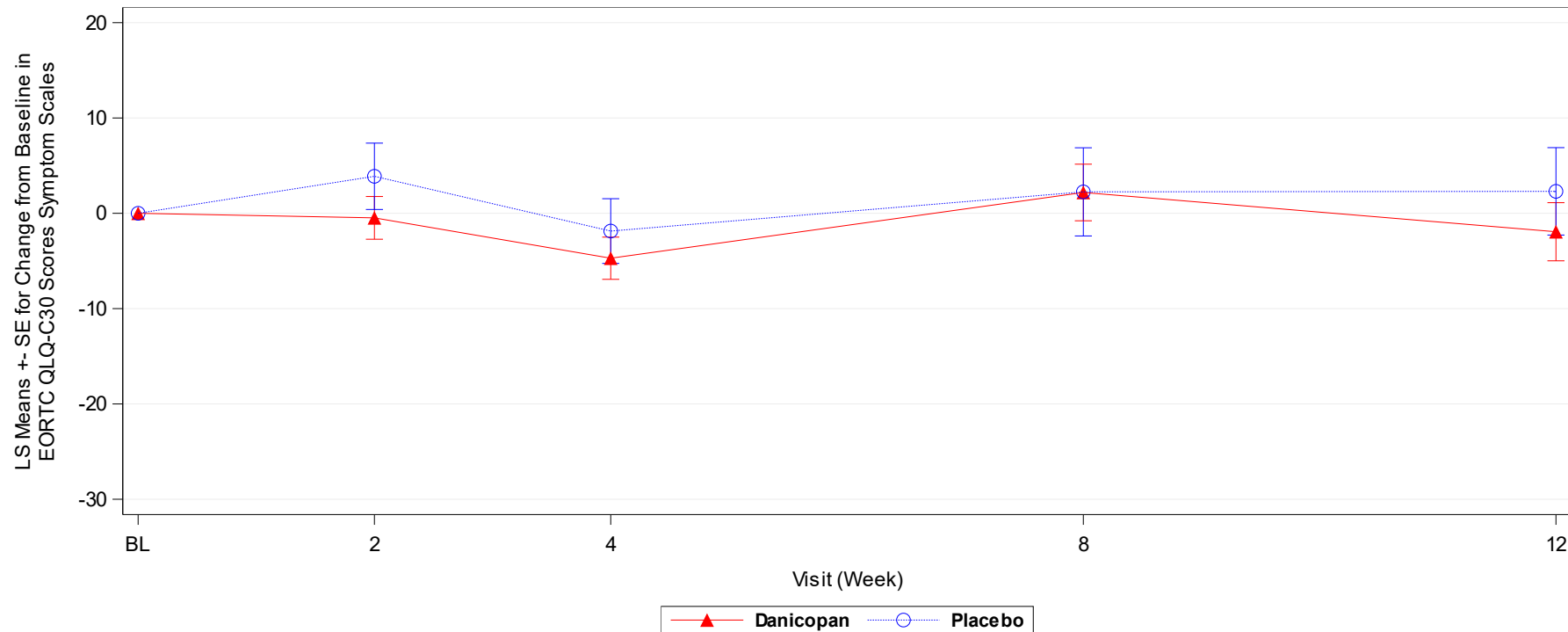
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Figure GERMANY.2.2.6.1
Plots of LS Means \pm SE for Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During 12-Week of Treatment Period 1
Modified Randomized Set

Appetite Loss



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Source: ADaM.ADSL, ADaM.ADQS

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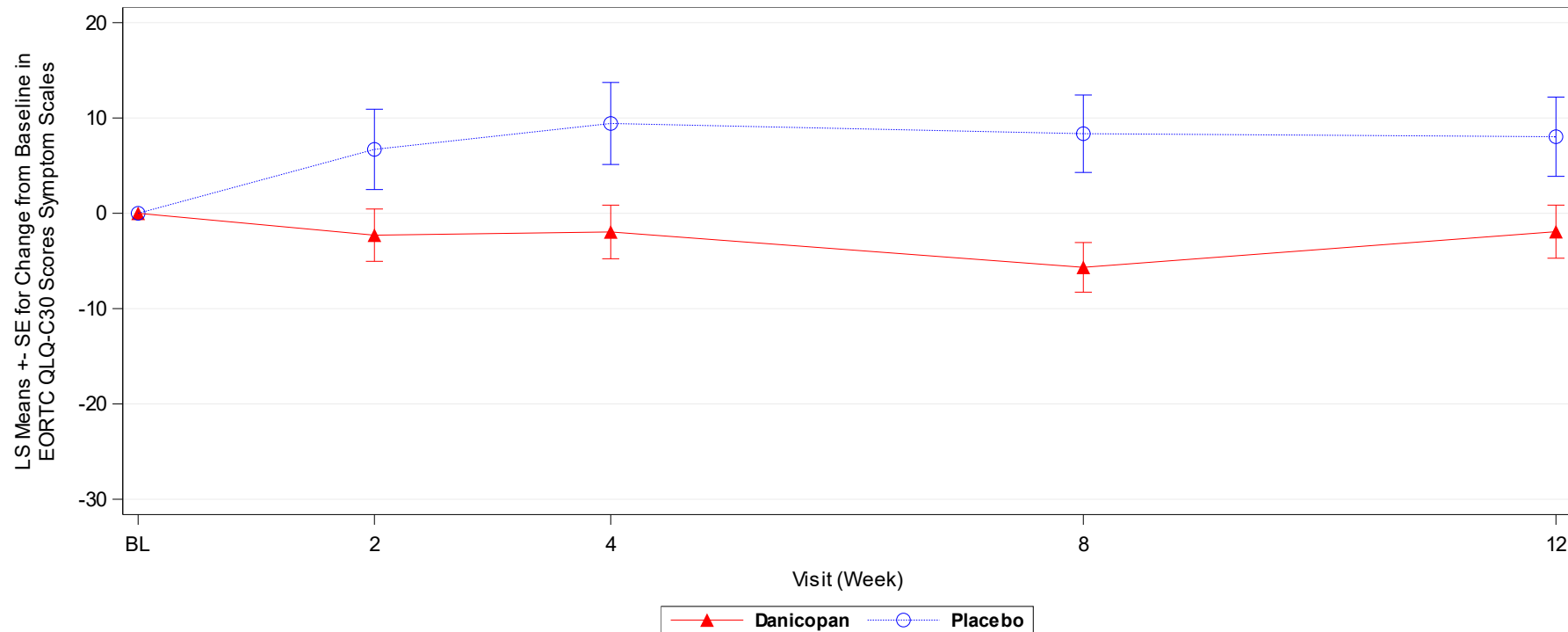
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Figure GERMANY.2.2.6.1
Plots of LS Means +- SE for Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During 12-Week of Treatment Period 1
Modified Randomized Set

Constipation



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Source: ADaM.ADSL, ADaM.ADQS

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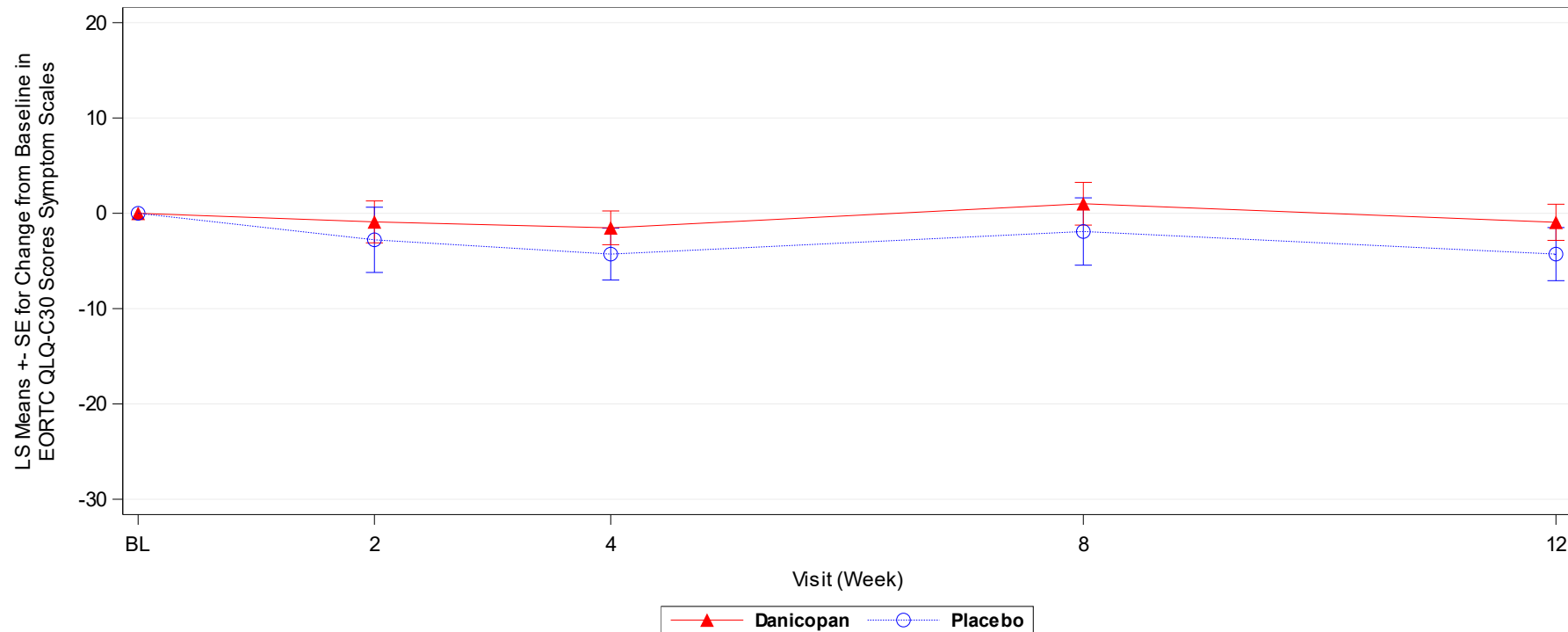
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Figure GERMANY.2.2.6.1
Plots of LS Means \pm SE for Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During 12-Week of Treatment Period 1
Modified Randomized Set

Diarrhea



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Source: ADaM.ADSL, ADaM.ADQS

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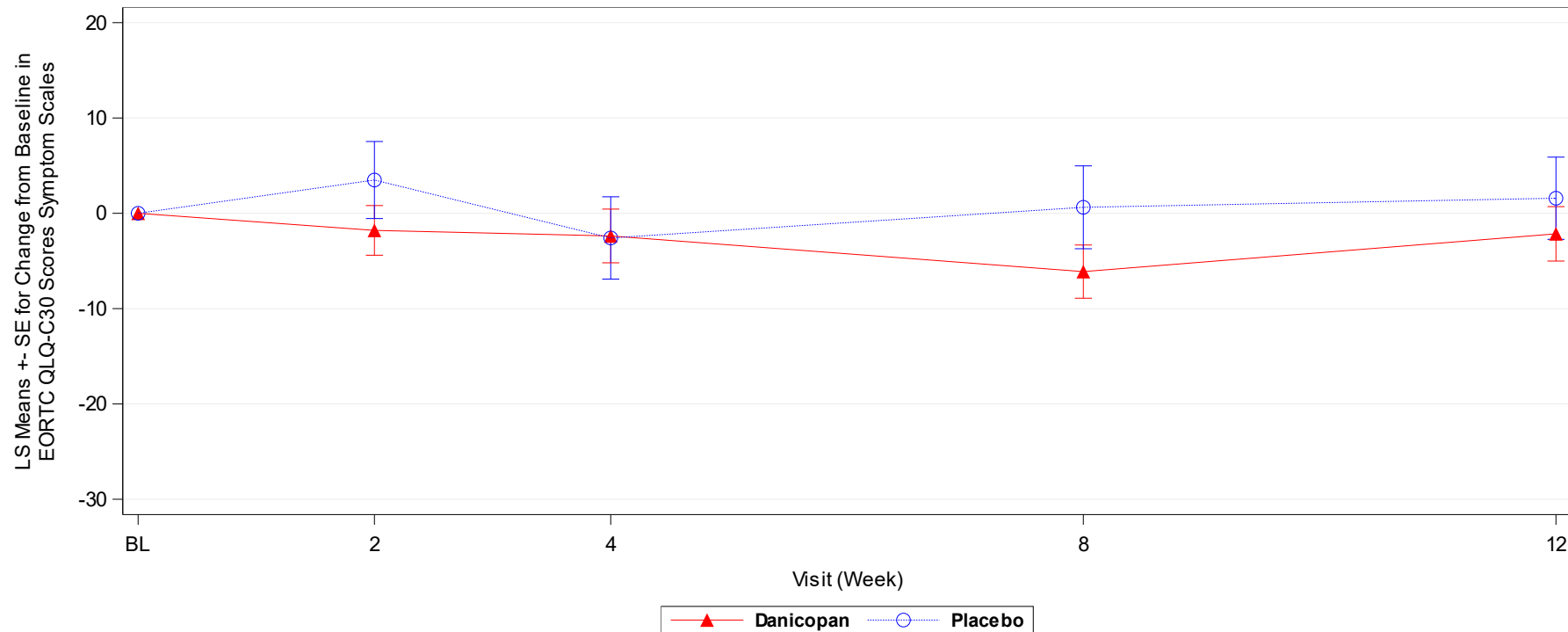
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Figure GERMANY.2.2.6.1
Plots of LS Means \pm SE for Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During 12-Week of Treatment Period 1
Modified Randomized Set

Financial Difficulties



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Source: ADaM.ADSL, ADaM.ADQS

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Data cutoff date of 31Mar2023

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.6 Symptomatik anhand des EORTC QLQ-C30

1.6.2 Veränderung der EORTC QLQ-C30 Symptomskalen im Vergleich zu Baseline

1.6.2.1 Subgruppenanalysen – Veränderung der EORTC QLQ-C30 Symptomskalen im Vergleich zu Baseline

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Fatigue

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean (SD)	39.61 (25.912)		38.27 (23.643)		39.24 (24.923)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 77.8		0.0, 100.0	
Week 2						
n	22	22	8	8		
Mean (SD)	27.78 (21.345)	-12.63 (22.300)	33.33 (11.878)	-1.39 (12.511)		
Median	33.33	-5.56	33.33	0.00		
Min, Max	0.0, 88.9	-66.7, 22.2	11.1, 55.6	-22.2, 11.1		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Fatigue

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	23	23	9	9		
Mean (SD)	26.09 (19.422)	-13.53 (22.462)	39.51 (20.869)	1.23 (12.963)		
Median	33.33	-11.11	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 22.2	11.1, 77.8	-11.1, 22.2		
Week 8						
n	19	19	7	7		
Mean (SD)	26.90 (17.100)	-8.19 (16.909)	20.63 (11.878)	-7.94 (15.335)		
Median	33.33	0.00	22.22	0.00		
Min, Max	0.0, 66.7	-44.4, 11.1	0.0, 33.3	-33.3, 11.1		

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Fatigue

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	9	9		
Mean (SD)	26.26 (20.443)	-13.64 (20.266)	37.04 (28.868)	-1.23 (20.370)		
Median	27.78	-11.11	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 22.2	0.0, 100.0	-44.4, 22.2		
Week 24						
n	21	21	9	9		
Mean (SD)	20.11 (23.732)	-19.58 (28.954)	20.99 (22.529)	-17.28 (25.526)		
Median	11.11	-22.22	22.22	-11.11		
Min, Max	0.0, 88.9	-77.8, 33.3	0.0, 55.6	-55.6, 22.2		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties. Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem. Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan. D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point. Baseline is defined as the last non-missing value prior to the first dose of study drug. Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Data cutoff date of 31Mar2023
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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Fatigue

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	8	8	29	29
Mean (SD)	21.69 (20.024)	-17.99 (23.433)	29.17 (31.949)	-11.11 (23.757)	23.75 (23.519)	-16.09 (23.304)
Median	22.22	-22.22	27.78	-11.11	22.22	-11.11
Min, Max	0.0, 77.8	-66.7, 22.2	0.0, 100.0	-44.4, 33.3	0.0, 100.0	-66.7, 33.3
Week 56						
n	17	17	5	5	22	22
Mean (SD)	30.72 (20.234)	-10.46 (28.456)	26.67 (12.669)	-15.56 (32.011)	29.80 (18.588)	-11.62 (28.581)
Median	33.33	-11.11	22.22	-22.22	33.33	-11.11
Min, Max	0.0, 66.7	-66.7, 44.4	11.1, 44.4	-55.6, 33.3	0.0, 66.7	-66.7, 44.4

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Fatigue

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	2	2	10	10
Mean (SD)	26.39 (13.197)	-27.78 (25.888)	33.33 (0.000)	-16.67 (23.570)	27.78 (12.001)	-25.56 (24.595)
Median	27.78	-16.67	33.33	-16.67	33.33	-16.67
Min, Max	0.0, 44.4	-66.7, 0.0	33.3, 33.3	-33.3, 0.0	0.0, 44.4	-66.7, 0.0
Week 88						
n	6	6	2	2	8	8
Mean (SD)	18.52 (13.456)	-31.48 (26.682)	16.67 (23.570)	-33.33 (0.000)	18.06 (14.472)	-31.94 (22.567)
Median	16.67	-38.89	16.67	-33.33	16.67	-33.33
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-33.3, -33.3	0.0, 33.3	-66.7, 0.0

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Fatigue

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	22.22 (NA)	-11.11 (NA)			22.22 (NA)	-11.11 (NA)
Median	22.22	-11.11			22.22	-11.11
Min, Max	22.2, 22.2	-11.1, -11.1			22.2, 22.2	-11.1, -11.1

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Nausea and Vomiting

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean (SD)	0.72 (3.475)		0.00 (0.000)		0.52 (2.946)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 16.7		0.0, 0.0		0.0, 16.7	
Week 2						
n	22	22	8	8		
Mean (SD)	0.00 (0.000)	-0.76 (3.553)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 0.0	-16.7, 0.0	0.0, 0.0	0.0, 0.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Nausea and Vomiting

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	23	23	9	9		
Mean (SD)	2.17 (5.739)	1.45 (6.950)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 16.7	-16.7, 16.7	0.0, 0.0	0.0, 0.0		
Week 8						
n	19	19	7	7		
Mean (SD)	1.75 (7.647)	0.88 (8.738)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-16.7, 33.3	0.0, 0.0	0.0, 0.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Nausea and Vomiting

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	9	9		
Mean (SD)	0.00 (0.000)	-0.76 (3.553)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 0.0	-16.7, 0.0	0.0, 0.0	0.0, 0.0		
Week 24						
n	21	21	9	9		
Mean (SD)	1.59 (7.274)	0.79 (8.294)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-16.7, 33.3	0.0, 0.0	0.0, 0.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Nausea and Vomiting

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	8	8	29	29
Mean (SD)	5.56 (10.971)	4.76 (10.728)	0.00 (0.000)	0.00 (0.000)	4.02 (9.611)	3.45 (9.322)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 0.0	0.0, 0.0	0.0, 33.3	0.0, 33.3
Week 56						
n	17	17	5	5	22	22
Mean (SD)	0.00 (0.000)	-0.98 (4.042)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	-0.76 (3.553)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	-16.7, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	-16.7, 0.0

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Nausea and Vomiting

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	2	2	10	10
Mean (SD)	0.00 (0.000)	-2.08 (5.893)	8.33 (11.785)	8.33 (11.785)	1.67 (5.270)	0.00 (7.857)
Median	0.00	0.00	8.33	8.33	0.00	0.00
Min, Max	0.0, 0.0	-16.7, 0.0	0.0, 16.7	0.0, 16.7	0.0, 16.7	-16.7, 16.7
Week 88						
n	6	6	2	2	8	8
Mean (SD)	0.00 (0.000)	0.00 (0.000)	8.33 (11.785)	8.33 (11.785)	2.08 (5.893)	2.08 (5.893)
Median	0.00	0.00	8.33	8.33	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 16.7	0.0, 16.7	0.0, 16.7	0.0, 16.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Nausea and Vomiting

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	0.00 (NA)			0.00 (NA)	0.00 (NA)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Pain

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean (SD)	14.49 (26.735)		11.11 (16.667)		13.54 (24.111)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 83.3		0.0, 33.3		0.0, 83.3	
Week 2						
n	22	22	8	8		
Mean (SD)	8.33 (22.272)	-6.82 (16.792)	14.58 (13.909)	2.08 (16.517)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 83.3	-66.7, 16.7	0.0, 33.3	-33.3, 16.7		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Pain

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	23	23	9	9		
Mean (SD)	10.87 (18.538)	-3.62 (21.879)	9.26 (14.699)	-1.85 (13.029)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 50.0	0.0, 33.3	-33.3, 16.7		
Week 8						
n	19	19	7	7		
Mean (SD)	8.77 (23.810)	0.00 (27.217)	4.76 (8.133)	-4.76 (20.893)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 16.7	-33.3, 16.7		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Pain

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	9	9		
Mean (SD)	4.55 (11.708)	-9.85 (24.484)	12.96 (16.197)	1.85 (24.216)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 50.0	-83.3, 16.7	0.0, 33.3	-33.3, 33.3		
Week 24						
n	21	21	9	9		
Mean (SD)	5.56 (10.971)	-9.52 (25.588)	7.41 (12.108)	-3.70 (18.215)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-83.3, 16.7	0.0, 33.3	-33.3, 16.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Pain

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	8	8	29	29
Mean (SD)	9.52 (20.798)	-5.56 (22.567)	18.75 (35.003)	10.42 (40.764)	12.07 (25.157)	-1.15 (28.844)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 83.3	-83.3, 16.7	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-83.3, 100.0
Week 56						
n	17	17	5	5	22	22
Mean (SD)	10.78 (22.002)	-7.84 (32.338)	33.33 (26.352)	20.00 (36.132)	15.91 (24.385)	-1.52 (34.468)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 66.7	-83.3, 66.7	0.0, 66.7	-16.7, 66.7	0.0, 66.7	-83.3, 66.7

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Pain

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	2	2	10	10
Mean (SD)	6.25 (12.400)	-14.58 (22.603)	16.67 (23.570)	0.00 (47.140)	8.33 (14.164)	-11.67 (26.117)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 33.3	-50.0, 0.0	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-50.0, 33.3
Week 88						
n	6	6	2	2	8	8
Mean (SD)	2.78 (6.804)	-13.89 (26.701)	25.00 (35.355)	8.33 (58.926)	8.33 (17.817)	-8.33 (33.333)
Median	0.00	0.00	25.00	8.33	0.00	0.00
Min, Max	0.0, 16.7	-66.7, 0.0	0.0, 50.0	-33.3, 50.0	0.0, 50.0	-66.7, 50.0

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Pain

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	0.00 (NA)			0.00 (NA)	0.00 (NA)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Dyspnoea

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean (SD)	26.09 (26.508)		29.63 (30.932)		27.08 (27.353)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	22	22	8	8		
Mean (SD)	15.15 (24.618)	-12.12 (21.932)	29.17 (21.362)	-4.17 (21.362)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	-33.3, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Dyspnoea

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	23	23	9	9		
Mean (SD)	8.70 (18.027)	-17.39 (22.178)	22.22 (23.570)	-7.41 (14.699)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-33.3, 0.0		
Week 8						
n	19	19	7	7		
Mean (SD)	14.04 (16.909)	-7.02 (17.843)	23.81 (16.265)	-14.29 (17.817)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 0.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Dyspnoea

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	9	9		
Mean (SD)	10.61 (15.891)	-13.64 (24.471)	18.52 (17.568)	-11.11 (28.868)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3		
Week 24						
n	21	21	9	9		
Mean (SD)	7.94 (17.965)	-17.46 (27.119)	14.81 (17.568)	-14.81 (24.216)		
Median	0.00	-33.33	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 33.3	-66.7, 0.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Dyspnoea

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	8	8	29	29
Mean (SD)	7.94 (14.548)	-19.05 (27.021)	8.33 (15.430)	-20.83 (24.801)	8.05 (14.516)	-19.54 (25.999)
Median	0.00	0.00	0.00	-16.67	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 33.3
Week 56						
n	17	17	5	5	22	22
Mean (SD)	11.76 (20.211)	-17.65 (29.149)	6.67 (14.907)	-6.67 (14.907)	10.61 (18.930)	-15.15 (26.681)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-66.7, 33.3

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Modified Randomized Set

Dyspnoea

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	2	2	10	10
Mean (SD)	4.17 (11.785)	-29.17 (27.817)	0.00 (0.000)	-33.33 (47.140)	3.33 (10.541)	-30.00 (29.187)
Median	0.00	-33.33	0.00	-33.33	0.00	-33.33
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 0.0	-66.7, 0.0	0.0, 33.3	-66.7, 0.0
Week 88						
n	6	6	2	2	8	8
Mean (SD)	5.56 (13.608)	-27.78 (25.092)	0.00 (0.000)	-33.33 (47.140)	4.17 (11.785)	-29.17 (27.817)
Median	0.00	-33.33	0.00	-33.33	0.00	-33.33
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 0.0	-66.7, 0.0	0.0, 33.3	-66.7, 0.0

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Dyspnoea

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	0.00 (NA)			0.00 (NA)	0.00 (NA)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Insomnia

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean (SD)	18.84 (24.259)		22.22 (28.868)		19.79 (25.201)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	22	22	8	8		
Mean (SD)	12.12 (24.224)	-6.06 (16.703)	12.50 (17.252)	-8.33 (23.570)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 33.3	-33.3, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Insomnia

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	23	23	9	9		
Mean (SD)	17.39 (28.194)	-1.45 (18.744)	11.11 (16.667)	-11.11 (23.570)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 33.3	-33.3, 33.3		
Week 8						
n	19	19	7	7		
Mean (SD)	14.04 (20.233)	-5.26 (20.071)	9.52 (16.265)	-4.76 (23.002)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 33.3	-33.3, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Insomnia

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	9	9		
Mean (SD)	13.64 (16.775)	-6.06 (19.616)	11.11 (16.667)	-11.11 (33.333)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-66.7, 33.3		
Week 24						
n	21	21	9	9		
Mean (SD)	9.52 (21.455)	-7.94 (25.614)	3.70 (11.111)	-18.52 (33.793)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 66.7	0.0, 33.3	-66.7, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Insomnia

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	8	8	29	29
Mean (SD)	15.87 (20.053)	-3.17 (23.345)	16.67 (35.635)	-8.33 (38.832)	16.09 (24.593)	-4.60 (27.781)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7
Week 56						
n	17	17	5	5	22	22
Mean (SD)	15.69 (20.809)	0.00 (28.868)	26.67 (27.889)	-6.67 (27.889)	18.18 (22.366)	-1.52 (28.129)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 66.7

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Insomnia

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	2	2	10	10
Mean (SD)	4.17 (11.785)	-4.17 (11.785)	33.33 (47.140)	-16.67 (70.711)	10.00 (22.498)	-6.67 (26.294)
Median	0.00	0.00	33.33	-16.67	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-66.7, 33.3
Week 88						
n	6	6	2	2	8	8
Mean (SD)	0.00 (0.000)	-5.56 (13.608)	16.67 (23.570)	-33.33 (47.140)	4.17 (11.785)	-12.50 (24.801)
Median	0.00	0.00	16.67	-33.33	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Insomnia

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	0.00 (NA)			0.00 (NA)	0.00 (NA)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Appetite Loss

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean (SD)	7.25 (14.058)		7.41 (14.699)		7.29 (14.000)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 33.3		0.0, 33.3		0.0, 33.3	
Week 2						
n	22	22	8	8		
Mean (SD)	9.09 (18.349)	1.52 (16.191)	16.67 (25.198)	8.33 (15.430)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	0.0, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
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Appetite Loss

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	23	23	9	9		
Mean (SD)	7.25 (14.058)	0.00 (10.050)	14.81 (33.793)	7.41 (22.222)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	0.0, 66.7		
Week 8						
n	19	19	7	7		
Mean (SD)	10.53 (15.919)	1.75 (13.488)	9.52 (16.265)	4.76 (12.599)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	0.0, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Appetite Loss

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	9	9		
Mean (SD)	7.58 (22.845)	1.52 (26.181)	18.52 (29.397)	11.11 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 66.7	0.0, 33.3		
Week 24						
n	21	21	9	9		
Mean (SD)	7.94 (23.345)	3.17 (27.698)	14.81 (24.216)	7.41 (14.699)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 66.7	0.0, 33.3		

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Modified Randomized Set

Appetite Loss

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	8	8	29	29
Mean (SD)	4.76 (11.952)	-1.59 (12.807)	8.33 (15.430)	0.00 (0.000)	5.75 (12.814)	-1.15 (10.848)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	0.0, 0.0	0.0, 33.3	-33.3, 33.3
Week 56						
n	17	17	5	5	22	22
Mean (SD)	7.84 (14.575)	1.96 (18.524)	6.67 (14.907)	0.00 (0.000)	7.58 (14.298)	1.52 (16.191)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	0.0, 0.0	0.0, 33.3	-33.3, 33.3

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Appetite Loss

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	2	2	10	10
Mean (SD)	0.00 (0.000)	-8.33 (15.430)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	-6.67 (14.055)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	-33.3, 0.0
Week 88						
n	6	6	2	2	8	8
Mean (SD)	5.56 (13.608)	0.00 (0.000)	16.67 (23.570)	16.67 (23.570)	8.33 (15.430)	4.17 (11.785)
Median	0.00	0.00	16.67	16.67	0.00	0.00
Min, Max	0.0, 33.3	0.0, 0.0	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Appetite Loss

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	0.00 (NA)			0.00 (NA)	0.00 (NA)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Constipation

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean (SD)	11.59 (19.092)		0.00 (0.000)		8.33 (16.933)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 0.0		0.0, 66.7	
Week 2						
n	22	22	8	8		
Mean (SD)	7.58 (14.298)	-4.55 (18.674)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 0.0	0.0, 0.0		

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Table GERMANY.2.5.4.2
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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Constipation

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	23	23	9	9		
Mean (SD)	5.80 (12.918)	-5.80 (21.678)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 0.0	0.0, 0.0		
Week 8						
n	19	19	7	7		
Mean (SD)	3.51 (10.510)	-5.26 (16.715)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 0.0	0.0, 0.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Constipation

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	9	9		
Mean (SD)	3.03 (9.808)	-9.09 (18.349)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 0.0	0.0, 0.0		
Week 24						
n	21	21	9	9		
Mean (SD)	1.59 (7.274)	-9.52 (21.455)	3.70 (11.111)	3.70 (11.111)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	0.0, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Constipation

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	8	8	29	29
Mean (SD)	4.76 (11.952)	-7.94 (23.345)	12.50 (24.801)	12.50 (24.801)	6.90 (16.377)	-2.30 (25.089)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	0.0, 66.7	0.0, 66.7	-66.7, 66.7
Week 56						
n	17	17	5	5	22	22
Mean (SD)	5.88 (13.098)	-7.84 (22.140)	0.00 (0.000)	0.00 (0.000)	4.55 (11.708)	-6.06 (19.616)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 0.0	0.0, 0.0	0.0, 33.3	-66.7, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Constipation

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	2	2	10	10
Mean (SD)	8.33 (15.430)	-8.33 (23.570)	0.00 (0.000)	0.00 (0.000)	6.67 (14.055)	-6.67 (21.082)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 0.0	0.0, 0.0	0.0, 33.3	-33.3, 33.3
Week 88						
n	6	6	2	2	8	8
Mean (SD)	11.11 (17.213)	-5.56 (13.608)	0.00 (0.000)	0.00 (0.000)	8.33 (15.430)	-4.17 (11.785)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 0.0	0.0, 0.0	0.0, 33.3	-33.3, 0.0

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Constipation

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	33.33 (NA)	0.00 (NA)			33.33 (NA)	0.00 (NA)
Median	33.33	0.00			33.33	0.00
Min, Max	33.3, 33.3	0.0, 0.0			33.3, 33.3	0.0, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Diarrhea

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean (SD)	4.35 (11.478)		0.00 (0.000)		3.13 (9.871)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 33.3		0.0, 0.0		0.0, 33.3	
Week 2						
n	23	23	8	8		
Mean (SD)	5.80 (16.368)	1.45 (6.950)	4.17 (11.785)	4.17 (11.785)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	0.0, 33.3	0.0, 33.3	0.0, 33.3		

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Diarrhea

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	23	23	9	9		
Mean (SD)	1.45 (6.950)	-2.90 (9.603)	3.70 (11.111)	3.70 (11.111)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	0.0, 33.3		
Week 8						
n	19	19	7	7		
Mean (SD)	5.26 (12.488)	0.00 (15.713)	4.76 (12.599)	4.76 (12.599)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	0.0, 33.3		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Diarrhea

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	9	9		
Mean (SD)	4.55 (11.708)	0.00 (14.548)	3.70 (11.111)	3.70 (11.111)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	0.0, 33.3		
Week 24						
n	21	21	9	9		
Mean (SD)	6.35 (13.412)	3.17 (14.548)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 0.0	0.0, 0.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Diarrhea

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	8	8	29	29
Mean (SD)	3.17 (10.026)	-1.59 (16.587)	4.17 (11.785)	4.17 (11.785)	3.45 (10.331)	0.00 (15.430)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3	-33.3, 33.3
Week 56						
n	17	17	5	5	22	22
Mean (SD)	3.92 (11.070)	1.96 (8.085)	0.00 (0.000)	0.00 (0.000)	3.03 (9.808)	1.52 (7.107)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 0.0	0.0, 0.0	0.0, 33.3	0.0, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Diarrhea

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	2	2	10	10
Mean (SD)	0.00 (0.000)	0.00 (0.000)	50.00 (70.711)	50.00 (70.711)	10.00 (31.623)	10.00 (31.623)
Median	0.00	0.00	50.00	50.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 100.0	0.0, 100.0	0.0, 100.0	0.0, 100.0
Week 88						
n	6	6	2	2	8	8
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Diarrhea

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	0.00 (NA)			0.00 (NA)	0.00 (NA)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Financial Difficulties

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean (SD)	21.74 (32.735)		22.22 (23.570)		21.88 (30.065)	
Median	0.00		33.33		0.00	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	23	23	8	8		
Mean (SD)	23.19 (33.986)	1.45 (18.744)	16.67 (17.817)	-4.17 (11.785)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 33.3	-33.3, 0.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Financial Difficulties

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	23	23	9	9		
Mean (SD)	15.94 (28.194)	-5.80 (19.207)	7.41 (14.699)	-14.81 (17.568)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-33.3, 0.0		
Week 8						
n	19	19	7	7		
Mean (SD)	12.28 (19.909)	-8.77 (21.779)	14.29 (26.227)	-9.52 (25.198)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-33.3, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Financial Difficulties

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	9	9		
Mean (SD)	21.21 (31.782)	-1.52 (19.182)	14.81 (24.216)	-7.41 (22.222)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 66.7	-33.3, 33.3		
Week 24						
n	21	21	9	9		
Mean (SD)	12.70 (24.667)	-9.52 (26.125)	18.52 (24.216)	-3.70 (20.031)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 66.7	-33.3, 33.3		

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Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	8	8	29	29
Mean (SD)	14.29 (24.881)	-9.52 (28.172)	20.83 (24.801)	-4.17 (21.362)	16.09 (24.593)	-8.05 (26.209)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-66.7, 33.3
Week 56						
n	17	17	5	5	22	22
Mean (SD)	13.73 (29.009)	-11.76 (23.396)	26.67 (27.889)	0.00 (23.570)	16.67 (28.637)	-9.09 (23.417)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-66.7, 33.3

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Financial Difficulties

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	2	2	10	10
Mean (SD)	8.33 (15.430)	-8.33 (15.430)	16.67 (23.570)	-16.67 (23.570)	10.00 (16.102)	-10.00 (16.102)
Median	0.00	0.00	16.67	-16.67	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 0.0
Week 88						
n	6	6	2	2	8	8
Mean (SD)	0.00 (0.000)	-11.11 (27.217)	16.67 (23.570)	-16.67 (23.570)	4.17 (11.785)	-12.50 (24.801)
Median	0.00	0.00	16.67	-16.67	0.00	0.00
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-66.7, 0.0

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Financial Difficulties

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	0.00 (NA)			0.00 (NA)	0.00 (NA)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Fatigue

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		15		49	
Mean (SD)	45.75 (28.131)		54.07 (27.496)		48.30 (27.921)	
Median	44.44		55.56		44.44	
Min, Max	0.0, 100.0		11.1, 100.0		0.0, 100.0	
Week 2						
n	33	33	17	15		
Mean (SD)	35.69 (27.883)	-10.10 (21.760)	50.33 (24.570)	-0.74 (26.383)		
Median	33.33	-11.11	44.44	0.00		
Min, Max	0.0, 100.0	-55.6, 33.3	11.1, 100.0	-44.4, 44.4		

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Fatigue

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	34	34	16	15		
Mean (SD)	29.25 (25.415)	-16.50 (25.311)	50.69 (23.992)	-0.74 (28.627)		
Median	27.78	-11.11	44.44	0.00		
Min, Max	0.0, 100.0	-55.6, 44.4	11.1, 100.0	-66.7, 44.4		
Week 8						
n	26	26	12	11		
Mean (SD)	30.34 (25.446)	-19.23 (27.855)	47.22 (16.497)	-7.07 (31.140)		
Median	33.33	-22.22	50.00	0.00		
Min, Max	0.0, 100.0	-66.7, 44.4	22.2, 77.8	-77.8, 44.4		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Fatigue

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	16	15		
Mean (SD)	28.15 (20.578)	-17.41 (21.774)	52.78 (27.666)	-2.96 (37.905)		
Median	33.33	-11.11	50.00	-11.11		
Min, Max	0.0, 66.7	-66.7, 22.2	11.1, 100.0	-77.8, 77.8		
Week 24						
n	28	28	15	14		
Mean (SD)	29.37 (23.073)	-15.87 (24.084)	44.44 (28.791)	-5.56 (33.968)		
Median	33.33	-11.11	33.33	-5.56		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-44.4, 77.8		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Fatigue

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	25	25	13	13	38	38
Mean (SD)	28.89 (25.660)	-14.67 (22.388)	50.43 (29.957)	2.56 (32.759)	36.26 (28.730)	-8.77 (27.236)
Median	22.22	-11.11	44.44	0.00	33.33	-5.56
Min, Max	0.0, 100.0	-77.8, 33.3	0.0, 100.0	-55.6, 66.7	0.0, 100.0	-77.8, 66.7
Week 56						
n	17	17	12	11	29	28
Mean (SD)	30.07 (26.283)	-11.11 (25.154)	37.04 (14.474)	-10.10 (26.036)	32.95 (22.119)	-10.71 (25.025)
Median	33.33	-11.11	33.33	-11.11	33.33	-11.11
Min, Max	0.0, 100.0	-66.7, 33.3	22.2, 66.7	-66.7, 44.4	0.0, 100.0	-66.7, 44.4

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Fatigue

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	10	9	23	22
Mean (SD)	25.64 (23.300)	-14.53 (16.595)	52.22 (23.454)	2.47 (28.748)	37.20 (26.508)	-7.58 (23.354)
Median	22.22	-11.11	50.00	0.00	33.33	-11.11
Min, Max	0.0, 66.7	-33.3, 22.2	22.2, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 66.7
Week 88						
n	8	8	6	6	14	14
Mean (SD)	22.22 (26.561)	-12.50 (22.567)	50.00 (16.851)	-7.41 (36.963)	34.13 (26.317)	-10.32 (28.400)
Median	11.11	-22.22	50.00	-5.56	33.33	-16.67
Min, Max	0.0, 66.7	-33.3, 33.3	33.3, 77.8	-66.7, 44.4	0.0, 77.8	-66.7, 44.4

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Fatigue

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	11.11 (19.245)	-29.63 (27.962)	27.78 (7.857)	-38.89 (54.997)	17.78 (16.851)	-33.33 (34.247)
Median	0.00	-33.33	27.78	-38.89	22.22	-33.33
Min, Max	0.0, 33.3	-55.6, 0.0	22.2, 33.3	-77.8, 0.0	0.0, 33.3	-77.8, 0.0

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Nausea and Vomiting

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		15		49	
Mean (SD)	3.92 (13.013)		10.00 (18.687)		5.78 (15.043)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	33	33	17	15		
Mean (SD)	6.57 (13.136)	2.53 (17.241)	10.78 (16.605)	0.00 (10.911)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 50.0	-50.0, 50.0	0.0, 50.0	-16.7, 16.7		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Nausea and Vomiting

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	34	34	16	15		
Mean (SD)	5.39 (11.401)	1.47 (15.001)	5.21 (10.035)	-4.44 (20.380)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-50.0, 33.3	0.0, 33.3	-66.7, 16.7		
Week 8						
n	26	26	12	11		
Mean (SD)	7.05 (17.747)	1.92 (19.623)	9.72 (15.006)	1.52 (25.226)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 83.3	-33.3, 83.3	0.0, 50.0	-66.7, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Nausea and Vomiting

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	16	15		
Mean (SD)	5.56 (11.015)	1.67 (13.384)	12.50 (23.174)	0.00 (24.398)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-50.0, 33.3	0.0, 83.3	-66.7, 50.0		
Week 24						
n	28	28	15	14		
Mean (SD)	4.17 (13.322)	0.00 (16.973)	5.56 (8.133)	0.00 (16.013)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-50.0, 66.7	0.0, 16.7	-33.3, 16.7		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Nausea and Vomiting

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	25	25	13	13	38	38
Mean (SD)	4.00 (7.265)	2.00 (7.328)	7.69 (14.618)	2.56 (17.803)	5.26 (10.329)	2.19 (11.735)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	-16.7, 16.7	0.0, 50.0	-16.7, 50.0	0.0, 50.0	-16.7, 50.0
Week 56						
n	17	17	12	11	29	28
Mean (SD)	0.98 (4.042)	-1.96 (10.004)	13.89 (28.278)	9.09 (28.249)	6.32 (19.114)	2.38 (19.623)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	-33.3, 16.7	0.0, 100.0	-33.3, 83.3	0.0, 100.0	-33.3, 83.3

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Nausea and Vomiting

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	10	9	23	22
Mean (SD)	1.28 (4.623)	0.00 (6.804)	6.67 (11.653)	1.85 (19.444)	3.62 (8.641)	0.76 (13.090)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	-16.7, 16.7	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3
Week 88						
n	8	8	6	6	14	14
Mean (SD)	2.08 (5.893)	0.00 (0.000)	8.33 (9.129)	0.00 (18.257)	4.76 (7.813)	0.00 (11.323)
Median	0.00	0.00	8.33	0.00	0.00	0.00
Min, Max	0.0, 16.7	0.0, 0.0	0.0, 16.7	-33.3, 16.7	0.0, 16.7	-33.3, 16.7

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Nausea and Vomiting

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	0.00 (0.000)	0.00 (0.000)	50.00 (23.570)	33.33 (0.000)	20.00 (29.814)	13.33 (18.257)
Median	0.00	0.00	50.00	33.33	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	33.3, 66.7	33.3, 33.3	0.0, 66.7	0.0, 33.3

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Pain

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		15		49	
Mean (SD)	14.71 (24.538)		26.67 (28.730)		18.37 (26.186)	
Median	0.00		16.67		0.00	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	33	33	16	14		
Mean (SD)	15.15 (24.067)	0.00 (17.678)	29.17 (23.174)	1.19 (21.147)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 66.7	-33.3, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Pain

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	34	34	16	15		
Mean (SD)	14.22 (23.971)	-0.49 (11.226)	27.08 (32.131)	2.22 (27.362)		
Median	0.00	0.00	25.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 83.3		
Week 8						
n	25	25	11	10		
Mean (SD)	15.33 (25.422)	2.67 (27.504)	13.64 (14.564)	-11.67 (15.811)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 33.3	-33.3, 0.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Pain

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	15	14		
Mean (SD)	16.11 (27.152)	1.11 (15.741)	31.11 (33.845)	3.57 (34.702)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 83.3		
Week 24						
n	28	28	15	14		
Mean (SD)	16.07 (29.565)	1.19 (20.752)	17.78 (24.774)	-7.14 (24.209)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-50.0, 66.7	0.0, 83.3	-66.7, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Pain

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	25	25	13	13	38	38
Mean (SD)	16.00 (25.676)	0.67 (17.665)	29.49 (34.125)	6.41 (28.495)	20.61 (29.109)	2.63 (21.757)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 83.3	-33.3, 33.3	0.0, 100.0	-33.3, 83.3	0.0, 100.0	-33.3, 83.3
Week 56						
n	17	17	12	11	29	28
Mean (SD)	23.53 (30.080)	3.92 (15.057)	22.22 (21.711)	0.00 (21.082)	22.99 (26.508)	2.38 (17.400)
Median	16.67	0.00	16.67	0.00	16.67	0.00
Min, Max	0.0, 100.0	-16.7, 33.3	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-33.3, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Pain

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	10	9	22	21
Mean (SD)	9.72 (19.408)	-5.56 (17.885)	23.33 (32.584)	11.11 (33.333)	15.91 (26.466)	1.59 (26.302)
Median	0.00	0.00	8.33	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 16.7	0.0, 100.0	-16.7, 83.3	0.0, 100.0	-33.3, 83.3
Week 88						
n	8	8	6	6	14	14
Mean (SD)	14.58 (24.296)	-4.17 (14.773)	25.00 (31.180)	19.44 (26.701)	19.05 (26.840)	5.95 (23.211)
Median	0.00	0.00	16.67	8.33	8.33	0.00
Min, Max	0.0, 66.7	-33.3, 16.7	0.0, 83.3	0.0, 66.7	0.0, 83.3	-33.3, 66.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Pain

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	11.11 (19.245)	-22.22 (38.490)	16.67 (0.000)	8.33 (11.785)	13.33 (13.944)	-10.00 (32.489)
Median	0.00	0.00	16.67	8.33	16.67	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	16.7, 16.7	0.0, 16.7	0.0, 33.3	-66.7, 16.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Dyspnoea

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		15		49	
Mean (SD)	44.12 (28.094)		31.11 (29.457)		40.14 (28.851)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	34	34	17	15		
Mean (SD)	27.45 (26.554)	-16.67 (23.570)	35.29 (29.978)	8.89 (29.457)		
Median	33.33	-16.67	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Dyspnoea

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	34	34	16	15		
Mean (SD)	18.63 (24.878)	-25.49 (27.289)	39.58 (36.956)	11.11 (39.171)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 66.7		
Week 8						
n	26	26	12	11		
Mean (SD)	21.79 (26.570)	-25.64 (25.487)	30.56 (33.207)	0.00 (42.164)		
Median	16.67	-33.33	33.33	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 100.0	-100.0, 66.7		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Dyspnoea

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	16	15		
Mean (SD)	21.11 (23.947)	-24.44 (24.656)	35.42 (25.730)	4.44 (45.192)		
Median	16.67	-33.33	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-100.0, 66.7		
Week 24						
n	28	28	15	14		
Mean (SD)	22.62 (24.094)	-20.24 (27.725)	15.56 (17.213)	-9.52 (27.514)		
Median	33.33	-33.33	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 33.3	-66.7, 33.3		

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Modified Randomized Set

Dyspnoea

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	25	25	13	13	38	38
Mean (SD)	25.33 (27.689)	-20.00 (21.517)	20.51 (16.879)	-7.69 (19.971)	23.68 (24.394)	-15.79 (21.556)
Median	33.33	-33.33	33.33	0.00	33.33	-16.67
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-66.7, 33.3
Week 56						
n	17	17	12	11	29	28
Mean (SD)	33.33 (31.180)	-9.80 (22.866)	13.89 (17.164)	-9.09 (30.151)	25.29 (27.682)	-9.52 (25.430)
Median	33.33	0.00	0.00	0.00	33.33	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 100.0	-66.7, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Dyspnoea

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	10	9	23	22
Mean (SD)	20.51 (25.598)	-25.64 (14.618)	23.33 (27.442)	-3.70 (30.932)	21.74 (25.837)	-16.67 (24.667)
Median	0.00	-33.33	16.67	0.00	0.00	-33.33
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3
Week 88						
n	8	8	6	6	14	14
Mean (SD)	8.33 (23.570)	-25.00 (23.570)	11.11 (17.213)	-11.11 (27.217)	9.52 (20.375)	-19.05 (25.198)
Median	0.00	-33.33	0.00	-16.67	0.00	-33.33
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-66.7, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Dyspnoea

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	0.00 (0.000)	-33.33 (33.333)	16.67 (23.570)	0.00 (0.000)	6.67 (14.907)	-20.00 (29.814)
Median	0.00	-33.33	16.67	0.00	0.00	0.00
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 33.3	0.0, 0.0	0.0, 33.3	-66.7, 0.0

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Insomnia

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		15		49	
Mean (SD)	31.37 (29.520)		33.33 (33.333)		31.97 (30.398)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	33	33	17	15		
Mean (SD)	28.28 (32.405)	-4.04 (21.663)	37.25 (35.123)	4.44 (35.337)		
Median	33.33	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 66.7		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Insomnia

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	34	34	16	15		
Mean (SD)	23.53 (33.363)	-7.84 (27.289)	33.33 (34.427)	0.00 (41.786)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-100.0, 66.7		
Week 8						
n	26	26	12	11		
Mean (SD)	12.82 (19.037)	-17.95 (27.049)	19.44 (30.011)	-9.09 (36.790)		
Median	0.00	-16.67	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-100.0, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Insomnia

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	16	15		
Mean (SD)	18.89 (28.612)	-12.22 (20.498)	41.67 (35.486)	8.89 (34.427)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 0.0	0.0, 100.0	-66.7, 66.7		
Week 24						
n	28	28	15	14		
Mean (SD)	19.05 (27.857)	-11.90 (30.381)	31.11 (23.458)	-2.38 (15.821)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 66.7	-33.3, 33.3		

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Insomnia

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	25	25	13	13	38	38
Mean (SD)	24.00 (32.660)	-9.33 (24.570)	30.77 (31.802)	-2.56 (41.859)	26.32 (32.101)	-7.02 (31.152)
Median	0.00	0.00	33.33	0.00	16.67	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-100.0, 66.7	0.0, 100.0	-100.0, 66.7
Week 56						
n	17	17	12	11	29	28
Mean (SD)	27.45 (31.700)	-7.84 (22.140)	41.67 (35.176)	6.06 (35.957)	33.33 (33.333)	-2.38 (28.586)
Median	33.33	0.00	33.33	0.00	33.33	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Insomnia

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	10	9	23	22
Mean (SD)	17.95 (22.008)	-17.95 (22.008)	40.00 (34.427)	0.00 (47.140)	27.54 (29.563)	-10.61 (34.712)
Median	0.00	0.00	33.33	0.00	33.33	0.00
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 100.0	-100.0, 66.7	0.0, 100.0	-100.0, 66.7
Week 88						
n	8	8	6	6	14	14
Mean (SD)	16.67 (25.198)	-16.67 (17.817)	50.00 (45.947)	11.11 (45.542)	30.95 (38.038)	-4.76 (34.237)
Median	0.00	-16.67	50.00	16.67	16.67	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Insomnia

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	11.11 (19.245)	-22.22 (38.490)	50.00 (70.711)	-16.67 (117.851)	26.67 (43.461)	-20.00 (64.979)
Median	0.00	0.00	50.00	-16.67	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 100.0	-100.0, 66.7	0.0, 100.0	-100.0, 66.7

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Appetite Loss

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		15		49	
Mean (SD)	9.80 (17.465)		24.44 (32.038)		14.29 (23.570)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 100.0		0.0, 100.0	
Week 2						
n	34	34	17	15		
Mean (SD)	10.78 (19.627)	0.98 (20.898)	21.57 (23.396)	-2.22 (15.258)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-33.3, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Appetite Loss

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	34	34	16	15		
Mean (SD)	4.90 (14.524)	-4.90 (16.682)	12.50 (23.960)	-11.11 (27.217)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-66.7, 33.3		
Week 8						
n	26	26	12	11		
Mean (SD)	15.38 (21.563)	2.56 (24.807)	13.89 (22.285)	-9.09 (33.635)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-100.0, 33.3		

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Appetite Loss

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	16	15		
Mean (SD)	6.67 (16.141)	-3.33 (23.732)	16.67 (29.814)	-6.67 (25.820)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 100.0	-66.7, 33.3		
Week 24						
n	28	28	15	14		
Mean (SD)	13.10 (22.842)	2.38 (29.991)	17.78 (24.774)	0.00 (13.074)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 100.0	0.0, 66.7	-33.3, 33.3		

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Appetite Loss

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	25	25	13	13	38	38
Mean (SD)	8.00 (14.530)	0.00 (19.245)	25.64 (30.894)	10.26 (34.385)	14.04 (22.769)	3.51 (25.456)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0
Week 56						
n	17	17	12	11	29	28
Mean (SD)	7.84 (14.575)	1.96 (21.955)	13.89 (30.011)	-3.03 (31.463)	10.34 (22.009)	0.00 (25.660)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Appetite Loss

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	10	9	23	22
Mean (SD)	5.13 (12.518)	-2.56 (9.245)	23.33 (22.498)	3.70 (26.058)	13.04 (19.434)	0.00 (17.817)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-33.3, 66.7
Week 88						
n	8	8	6	6	14	14
Mean (SD)	8.33 (15.430)	0.00 (17.817)	16.67 (18.257)	-5.56 (32.773)	11.90 (16.575)	-2.38 (24.335)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Appetite Loss

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	0.00 (0.000)	0.00 (0.000)	66.67 (0.000)	16.67 (23.570)	26.67 (36.515)	6.67 (14.907)
Median	0.00	0.00	66.67	16.67	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	0.0, 33.3	0.0, 66.7	0.0, 33.3

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Constipation

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		15		49	
Mean (SD)	14.71 (26.197)		17.78 (21.331)		15.65 (24.629)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	33	33	17	15		
Mean (SD)	11.11 (27.217)	-4.04 (19.996)	25.49 (34.419)	8.89 (32.038)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Constipation

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	34	34	16	15		
Mean (SD)	12.75 (27.235)	-1.96 (21.620)	29.17 (31.914)	13.33 (30.342)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-33.3, 66.7		
Week 8						
n	26	26	12	11		
Mean (SD)	7.69 (17.147)	-7.69 (23.679)	25.00 (35.176)	12.12 (16.817)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	0.0, 33.3		

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Constipation

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	16	15		
Mean (SD)	14.44 (29.921)	0.00 (21.442)	27.08 (36.956)	11.11 (27.217)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 66.7		
Week 24						
n	28	28	15	14		
Mean (SD)	10.71 (24.094)	-3.57 (18.898)	24.44 (29.457)	7.14 (26.726)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 66.7		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Constipation

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	25	25	13	13	38	38
Mean (SD)	8.00 (17.427)	-9.33 (20.458)	17.95 (22.008)	0.00 (19.245)	11.40 (19.415)	-6.14 (20.291)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-66.7, 33.3
Week 56						
n	17	17	12	11	29	28
Mean (SD)	13.73 (29.009)	-7.84 (18.743)	16.67 (22.473)	0.00 (14.907)	14.94 (26.105)	-4.76 (17.484)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 0.0	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-66.7, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Constipation

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	10	9	23	22
Mean (SD)	15.38 (29.235)	-7.69 (19.971)	13.33 (17.213)	3.70 (20.031)	14.49 (24.259)	-3.03 (20.339)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 33.3
Week 88						
n	8	8	6	6	14	14
Mean (SD)	16.67 (17.817)	-4.17 (27.817)	33.33 (51.640)	22.22 (50.185)	23.81 (35.635)	7.14 (39.610)
Median	16.67	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Constipation

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	11.11 (19.245)	-11.11 (19.245)	33.33 (47.140)	0.00 (47.140)	20.00 (29.814)	-6.67 (27.889)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Diarrhea

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		15		49	
Mean (SD)	5.88 (19.193)		20.00 (24.560)		10.20 (21.735)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	33	33	17	15		
Mean (SD)	6.06 (19.462)	0.00 (22.048)	5.88 (17.620)	-13.33 (24.560)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 100.0	0.0, 66.7	-66.7, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Diarrhea

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	34	34	16	15		
Mean (SD)	7.84 (16.532)	1.96 (24.538)	4.17 (11.386)	-15.56 (21.331)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-100.0, 66.7	0.0, 33.3	-66.7, 0.0		
Week 8						
n	26	26	12	11		
Mean (SD)	8.97 (17.783)	3.85 (21.760)	8.33 (20.719)	-15.15 (17.408)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 66.7	0.0, 66.7	-33.3, 0.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Diarrhea

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	16	15		
Mean (SD)	6.67 (13.561)	0.00 (17.508)	4.17 (16.667)	-15.56 (33.014)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-66.7, 66.7		
Week 24						
n	28	28	15	14		
Mean (SD)	4.76 (11.878)	-1.19 (16.929)	6.67 (13.801)	-11.90 (21.111)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 0.0		

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Diarrhea

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	25	25	13	13	38	38
Mean (SD)	6.67 (13.608)	-1.33 (24.495)	10.26 (16.013)	-10.26 (28.495)	7.89 (14.362)	-4.39 (25.902)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-100.0, 33.3
Week 56						
n	17	17	12	11	29	28
Mean (SD)	9.80 (15.656)	1.96 (29.978)	11.11 (16.412)	-15.15 (27.340)	10.34 (15.694)	-4.76 (29.696)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-100.0, 33.3

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Diarrhea

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	10	9	22	21
Mean (SD)	0.00 (0.000)	-2.78 (9.623)	20.00 (23.307)	-3.70 (20.031)	9.09 (18.349)	-3.17 (14.548)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3
Week 88						
n	8	8	6	6	14	14
Mean (SD)	4.17 (11.785)	4.17 (11.785)	11.11 (17.213)	-11.11 (34.427)	7.14 (14.194)	-2.38 (24.335)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3

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Diarrhea

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Financial Difficulties

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		15		49	
Mean (SD)	19.61 (29.721)		24.44 (36.659)		21.09 (31.691)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	34	34	17	15		
Mean (SD)	14.71 (26.197)	-4.90 (11.983)	27.45 (39.503)	6.67 (33.806)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 0.0	0.0, 100.0	-66.7, 100.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Financial Difficulties

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	34	34	16	15		
Mean (SD)	18.63 (26.197)	-0.98 (20.898)	25.00 (39.441)	2.22 (34.427)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 100.0		
Week 8						
n	26	26	12	11		
Mean (SD)	12.82 (23.242)	-5.13 (22.494)	13.89 (22.285)	6.06 (13.484)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 66.7	0.0, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Financial Difficulties

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	16	15		
Mean (SD)	15.56 (22.715)	-3.33 (16.022)	27.08 (40.768)	4.44 (37.515)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 100.0		
Week 24						
n	28	28	15	14		
Mean (SD)	21.43 (31.706)	2.38 (23.880)	24.44 (40.760)	0.00 (34.592)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 100.0	0.0, 100.0	-66.7, 100.0		

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Modified Randomized Set

Financial Difficulties

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	25	25	13	13	38	38
Mean (SD)	20.00 (28.868)	-1.33 (17.951)	20.51 (37.363)	-7.69 (43.363)	20.18 (31.518)	-3.51 (28.778)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-100.0, 100.0	0.0, 100.0	-100.0, 100.0
Week 56						
n	17	17	12	11	29	28
Mean (SD)	11.76 (16.420)	-5.88 (13.098)	25.00 (37.939)	-3.03 (45.837)	17.24 (27.632)	-4.76 (29.696)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 100.0	-66.7, 100.0	0.0, 100.0	-66.7, 100.0

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Table GERMANY.2.5.4.2
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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Financial Difficulties

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	10	9	22	21
Mean (SD)	8.33 (15.076)	-11.11 (29.588)	10.00 (22.498)	-14.81 (24.216)	9.09 (18.349)	-12.70 (26.825)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-66.7, 0.0	0.0, 66.7	-66.7, 33.3
Week 88						
n	8	8	6	6	14	14
Mean (SD)	16.67 (17.817)	-8.33 (15.430)	5.56 (13.608)	-27.78 (32.773)	11.90 (16.575)	-16.67 (25.318)
Median	16.67	0.00	0.00	-16.67	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0

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Table GERMANY.2.5.4.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Financial Difficulties

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	0.00 (0.000)	-22.22 (38.490)	16.67 (23.570)	-66.67 (0.000)	6.67 (14.907)	-40.00 (36.515)
Median	0.00	0.00	16.67	-66.67	0.00	-66.67
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 33.3	-66.7, -66.7	0.0, 33.3	-66.7, 0.0

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Fatigue

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	41		18		59	
Mean (SD)	46.88 (28.275)		52.47 (28.214)		48.59 (28.132)	
Median	44.44		50.00		44.44	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	40	40	20	18		
Mean (SD)	32.22 (25.881)	-15.28 (22.741)	46.11 (24.256)	-4.32 (22.911)		
Median	33.33	-11.11	38.89	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	11.1, 100.0	-44.4, 44.4		

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Modified Randomized Set

Fatigue

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	41	41	19	18		
Mean (SD)	28.18 (24.101)	-18.70 (24.775)	46.20 (24.641)	-4.32 (24.739)		
Median	22.22	-11.11	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 44.4	11.1, 100.0	-66.7, 44.4		
Week 8						
n	32	32	14	13		
Mean (SD)	26.04 (19.878)	-21.53 (22.567)	37.30 (19.298)	-13.68 (24.069)		
Median	33.33	-22.22	33.33	-11.11		
Min, Max	0.0, 66.7	-66.7, 11.1	0.0, 66.7	-77.8, 11.1		

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Fatigue

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	39	39	19	18		
Mean (SD)	26.78 (21.587)	-19.09 (21.166)	46.78 (29.532)	-7.41 (31.886)		
Median	33.33	-22.22	44.44	-5.56		
Min, Max	0.0, 66.7	-66.7, 11.1	0.0, 100.0	-77.8, 77.8		
Week 24						
n	36	36	18	17		
Mean (SD)	24.07 (24.560)	-21.60 (26.818)	34.57 (32.313)	-15.03 (34.233)		
Median	22.22	-22.22	33.33	-22.22		
Min, Max	0.0, 100.0	-77.8, 33.3	0.0, 100.0	-55.6, 77.8		

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Fatigue

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	33	33	16	16	49	49
Mean (SD)	25.25 (25.348)	-19.87 (23.856)	37.50 (28.939)	-9.72 (26.566)	29.25 (26.902)	-16.55 (24.961)
Median	22.22	-11.11	33.33	-5.56	33.33	-11.11
Min, Max	0.0, 100.0	-77.8, 33.3	0.0, 100.0	-55.6, 44.4	0.0, 100.0	-77.8, 44.4
Week 56						
n	23	23	15	14	38	37
Mean (SD)	29.47 (24.067)	-15.46 (26.323)	34.07 (14.828)	-11.90 (28.732)	31.29 (20.804)	-14.11 (26.919)
Median	33.33	-11.11	33.33	-11.11	33.33	-11.11
Min, Max	0.0, 100.0	-66.7, 33.3	11.1, 66.7	-66.7, 44.4	0.0, 100.0	-66.7, 44.4

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Run Date: 2024-04-26T15:48:25

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Fatigue

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	15	15	10	9	25	24
Mean (SD)	25.93 (19.994)	-22.22 (23.757)	45.56 (16.932)	-4.94 (15.822)	33.78 (20.905)	-15.74 (22.443)
Median	33.33	-22.22	38.89	0.00	33.33	-11.11
Min, Max	0.0, 66.7	-66.7, 22.2	22.2, 66.7	-33.3, 22.2	0.0, 66.7	-66.7, 22.2
Week 88						
n	8	8	6	6	14	14
Mean (SD)	18.06 (22.954)	-25.00 (24.307)	37.04 (20.688)	-20.37 (27.592)	26.19 (23.310)	-23.02 (24.832)
Median	11.11	-27.78	38.89	-16.67	27.78	-22.22
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 55.6	-66.7, 11.1	0.0, 66.7	-66.7, 11.1

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Fatigue
< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	7.41 (12.830)	-14.81 (16.973)	27.78 (7.857)	-38.89 (54.997)	15.56 (14.907)	-24.44 (32.773)
Median	0.00	-11.11	27.78	-38.89	22.22	-11.11
Min, Max	0.0, 22.2	-33.3, 0.0	22.2, 33.3	-77.8, 0.0	0.0, 33.3	-77.8, 0.0

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Nausea and Vomiting

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	41		18		59	
Mean (SD)	3.66 (12.083)		8.33 (17.386)		5.08 (13.929)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	40	40	20	18		
Mean (SD)	4.17 (9.806)	0.42 (13.861)	9.17 (15.742)	0.00 (9.901)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 50.0	-50.0, 50.0	0.0, 50.0	-16.7, 16.7		

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Nausea and Vomiting

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	41	41	19	18		
Mean (SD)	4.47 (9.877)	0.81 (13.412)	4.39 (9.366)	-3.70 (18.573)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-50.0, 33.3	0.0, 33.3	-66.7, 16.7		
Week 8						
n	32	32	14	13		
Mean (SD)	6.77 (16.859)	2.08 (18.813)	7.14 (14.194)	0.00 (22.567)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 83.3	-33.3, 83.3	0.0, 50.0	-66.7, 33.3		

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Nausea and Vomiting

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	39	39	19	18		
Mean (SD)	3.42 (8.702)	0.00 (10.815)	9.65 (21.742)	-0.93 (21.747)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-50.0, 33.3	0.0, 83.3	-66.7, 50.0		
Week 24						
n	36	36	18	17		
Mean (SD)	4.17 (12.833)	0.46 (16.177)	4.63 (7.681)	0.00 (14.434)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-50.0, 66.7	0.0, 16.7	-33.3, 16.7		

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Nausea and Vomiting

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	33	33	16	16	49	49
Mean (SD)	6.06 (10.050)	4.04 (10.232)	3.13 (6.719)	-1.04 (9.562)	5.10 (9.131)	2.38 (10.206)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-16.7, 33.3	0.0, 16.7	-16.7, 16.7	0.0, 33.3	-16.7, 33.3
Week 56						
n	23	23	15	14	38	37
Mean (SD)	0.72 (3.475)	-2.17 (9.135)	11.11 (25.717)	7.14 (25.076)	4.82 (16.849)	1.35 (17.293)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	-33.3, 16.7	0.0, 100.0	-33.3, 83.3	0.0, 100.0	-33.3, 83.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Nausea and Vomiting

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	15	15	10	9	25	24
Mean (SD)	1.11 (4.303)	-1.11 (7.629)	3.33 (7.027)	-1.85 (15.466)	2.00 (5.528)	-1.39 (10.898)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	-16.7, 16.7	0.0, 16.7	-33.3, 16.7	0.0, 16.7	-33.3, 16.7
Week 88						
n	8	8	6	6	14	14
Mean (SD)	2.08 (5.893)	0.00 (0.000)	8.33 (9.129)	0.00 (18.257)	4.76 (7.813)	0.00 (11.323)
Median	0.00	0.00	8.33	0.00	0.00	0.00
Min, Max	0.0, 16.7	0.0, 0.0	0.0, 16.7	-33.3, 16.7	0.0, 16.7	-33.3, 16.7

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Nausea and Vomiting

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	0.00 (0.000)	0.00 (0.000)	50.00 (23.570)	33.33 (0.000)	20.00 (29.814)	13.33 (18.257)
Median	0.00	0.00	50.00	33.33	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	33.3, 66.7	33.3, 33.3	0.0, 66.7	0.0, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Pain

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	41		18		59	
Mean (SD)	15.85 (25.263)		23.15 (28.087)		18.08 (26.131)	
Median	0.00		16.67		0.00	
Min, Max	0.0, 83.3		0.0, 100.0		0.0, 100.0	
Week 2						
n	40	40	20	18		
Mean (SD)	11.25 (20.806)	-5.00 (15.191)	25.00 (23.258)	0.93 (20.189)		
Median	0.00	0.00	25.00	0.00		
Min, Max	0.0, 83.3	-66.7, 16.7	0.0, 66.7	-33.3, 33.3		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Pain

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	41	41	19	18		
Mean (SD)	12.60 (19.643)	-3.25 (17.167)	21.05 (31.349)	-0.93 (25.866)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-33.3, 83.3		
Week 8						
n	32	32	14	13		
Mean (SD)	15.10 (27.225)	2.60 (29.666)	9.52 (12.599)	-10.26 (17.398)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 33.3	-33.3, 16.7		

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Modified Randomized Set

Pain

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	39	39	19	18		
Mean (SD)	11.97 (20.925)	-3.42 (22.679)	24.56 (32.569)	0.93 (32.576)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 66.7	-83.3, 33.3	0.0, 100.0	-33.3, 83.3		
Week 24						
n	36	36	18	17		
Mean (SD)	10.65 (22.237)	-5.09 (26.071)	14.81 (23.493)	-6.86 (23.614)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-83.3, 66.7	0.0, 83.3	-66.7, 33.3		

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Pain

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	33	33	16	16	49	49
Mean (SD)	12.12 (22.925)	-3.54 (21.553)	17.71 (26.154)	-2.08 (17.078)	13.95 (23.899)	-3.06 (20.033)
Median	0.00	0.00	8.33	0.00	0.00	0.00
Min, Max	0.0, 83.3	-83.3, 33.3	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-83.3, 33.3
Week 56						
n	23	23	15	14	38	37
Mean (SD)	13.77 (22.277)	-6.52 (25.985)	24.44 (20.766)	4.76 (21.111)	17.98 (22.050)	-2.25 (24.584)
Median	0.00	0.00	16.67	0.00	8.33	0.00
Min, Max	0.0, 66.7	-83.3, 33.3	0.0, 66.7	-33.3, 50.0	0.0, 66.7	-83.3, 50.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Pain

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	14	14	10	9	24	23
Mean (SD)	4.76 (10.187)	-11.90 (20.073)	13.33 (18.922)	-1.85 (22.737)	8.33 (14.744)	-7.97 (21.243)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-50.0, 0.0	0.0, 50.0	-33.3, 50.0	0.0, 50.0	-50.0, 50.0
Week 88						
n	8	8	6	6	14	14
Mean (SD)	6.25 (12.400)	-10.42 (23.465)	11.11 (13.608)	2.78 (22.153)	8.33 (12.659)	-4.76 (23.046)
Median	0.00	0.00	8.33	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-66.7, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Pain

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	0.00 (0.000)	0.00 (0.000)	16.67 (0.000)	8.33 (11.785)	6.67 (9.129)	3.33 (7.454)
Median	0.00	0.00	16.67	8.33	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	16.7, 16.7	0.0, 16.7	0.0, 16.7	0.0, 16.7

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Dyspnoea

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	41		18		59	
Mean (SD)	35.77 (31.083)		31.48 (31.253)		34.46 (30.928)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	40	40	20	18		
Mean (SD)	20.83 (26.887)	-15.83 (23.856)	30.00 (28.408)	1.85 (29.087)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

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Modified Randomized Set

Dyspnoea

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	41	41	19	18		
Mean (SD)	12.20 (20.758)	-23.58 (26.081)	31.58 (35.959)	1.85 (36.999)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-66.7, 66.7		
Week 8						
n	32	32	14	13		
Mean (SD)	13.54 (18.664)	-20.83 (25.045)	23.81 (30.462)	-10.26 (36.980)		
Median	0.00	-33.33	16.67	0.00		
Min, Max	0.0, 66.7	-100.0, 33.3	0.0, 100.0	-100.0, 66.7		

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Modified Randomized Set

Dyspnoea

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	39	39	19	18		
Mean (SD)	15.38 (21.421)	-20.51 (26.063)	28.07 (25.491)	-3.70 (44.116)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-100.0, 66.7		
Week 24						
n	36	36	18	17		
Mean (SD)	12.96 (21.496)	-21.30 (28.898)	12.96 (16.721)	-13.73 (29.009)		
Median	0.00	-33.33	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 33.3	-66.7, 33.3		

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Dyspnoea

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	33	33	16	16	49	49
Mean (SD)	16.16 (25.168)	-20.20 (24.916)	16.67 (17.213)	-12.50 (23.960)	16.33 (22.692)	-17.69 (24.629)
Median	0.00	-33.33	16.67	0.00	0.00	-33.33
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 100.0	-66.7, 33.3
Week 56						
n	23	23	15	14	38	37
Mean (SD)	20.29 (26.091)	-15.94 (24.349)	11.11 (16.265)	-11.90 (24.832)	16.67 (22.924)	-14.41 (24.268)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-66.7, 33.3

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Dyspnoea

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	15	15	10	9	25	24
Mean (SD)	13.33 (21.082)	-28.89 (17.213)	16.67 (23.570)	-14.81 (33.793)	14.67 (21.688)	-23.61 (25.020)
Median	0.00	-33.33	0.00	-33.33	0.00	-33.33
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-66.7, 33.3
Week 88						
n	8	8	6	6	14	14
Mean (SD)	8.33 (23.570)	-20.83 (24.801)	11.11 (17.213)	-16.67 (34.960)	9.52 (20.375)	-19.05 (28.388)
Median	0.00	-16.67	0.00	-16.67	0.00	-16.67
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-66.7, 33.3

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Dyspnoea

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	0.00 (0.000)	-11.11 (19.245)	16.67 (23.570)	0.00 (0.000)	6.67 (14.907)	-6.67 (14.907)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 33.3	0.0, 0.0	0.0, 33.3	-33.3, 0.0

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Insomnia

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	41		18		59	
Mean (SD)	29.27 (29.055)		37.04 (32.113)		31.64 (29.958)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	40	40	20	18		
Mean (SD)	20.00 (30.005)	-9.17 (18.471)	33.33 (34.199)	-3.70 (34.087)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 66.7		

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Modified Randomized Set

Insomnia
< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	41	41	19	18		
Mean (SD)	22.76 (32.863)	-6.50 (24.966)	31.58 (32.344)	-5.56 (40.016)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-100.0, 66.7		
Week 8						
n	32	32	14	13		
Mean (SD)	10.42 (15.698)	-19.79 (23.736)	16.67 (28.495)	-15.38 (32.247)		
Median	0.00	-33.33	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 100.0	-100.0, 33.3		

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Modified Randomized Set

Insomnia

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	39	39	19	18		
Mean (SD)	17.09 (25.215)	-11.97 (19.477)	36.84 (34.951)	0.00 (37.920)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 0.0	0.0, 100.0	-66.7, 66.7		
Week 24						
n	36	36	18	17		
Mean (SD)	14.81 (26.956)	-12.96 (25.545)	25.93 (24.403)	-11.76 (26.197)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 66.7	-66.7, 33.3		

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Insomnia

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	33	33	16	16	49	49
Mean (SD)	20.20 (29.976)	-10.10 (22.799)	25.00 (31.032)	-12.50 (40.139)	21.77 (30.086)	-10.88 (29.177)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-100.0, 66.7	0.0, 100.0	-100.0, 66.7
Week 56						
n	23	23	15	14	38	37
Mean (SD)	20.29 (29.711)	-8.70 (20.640)	35.56 (34.427)	-2.38 (33.242)	26.32 (32.101)	-6.31 (25.865)
Median	0.00	0.00	33.33	0.00	33.33	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Insomnia

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	15	15	10	9	25	24
Mean (SD)	13.33 (21.082)	-13.33 (21.082)	33.33 (35.136)	-14.81 (44.444)	21.33 (28.674)	-13.89 (30.954)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 100.0	-100.0, 33.3	0.0, 100.0	-100.0, 33.3
Week 88						
n	8	8	6	6	14	14
Mean (SD)	12.50 (24.801)	-8.33 (15.430)	50.00 (45.947)	0.00 (55.777)	28.57 (38.911)	-4.76 (36.648)
Median	0.00	0.00	50.00	16.67	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Insomnia

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	11.11 (19.245)	0.00 (0.000)	50.00 (70.711)	-16.67 (117.851)	26.67 (43.461)	-6.67 (59.628)
Median	0.00	0.00	50.00	-16.67	0.00	0.00
Min, Max	0.0, 33.3	0.0, 0.0	0.0, 100.0	-100.0, 66.7	0.0, 100.0	-100.0, 66.7

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Modified Randomized Set

Appetite Loss

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	41		18		59	
Mean (SD)	7.32 (13.969)		22.22 (30.250)		11.86 (21.229)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 33.3		0.0, 100.0		0.0, 100.0	
Week 2						
n	40	40	20	18		
Mean (SD)	7.50 (15.991)	0.00 (18.490)	21.67 (24.839)	0.00 (16.169)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-33.3, 33.3		

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Modified Randomized Set

Appetite Loss

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	41	41	19	18		
Mean (SD)	4.88 (11.928)	-2.44 (15.617)	15.79 (30.161)	-5.56 (30.785)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-66.7, 66.7		
Week 8						
n	32	32	14	13		
Mean (SD)	9.38 (15.227)	0.00 (16.933)	11.90 (21.111)	-7.69 (30.894)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-100.0, 33.3		

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Appetite Loss

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	39	39	19	18		
Mean (SD)	5.13 (17.997)	-1.71 (22.876)	17.54 (30.161)	-3.70 (25.280)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-66.7, 33.3		
Week 24						
n	36	36	18	17		
Mean (SD)	10.19 (24.969)	3.70 (28.483)	16.67 (23.570)	0.00 (11.785)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 66.7	-33.3, 33.3		

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Appetite Loss

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	33	33	16	16	49	49
Mean (SD)	3.03 (9.731)	-3.03 (12.812)	16.67 (21.082)	2.08 (19.124)	7.48 (15.613)	-1.36 (15.152)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3
Week 56						
n	23	23	15	14	38	37
Mean (SD)	5.80 (12.918)	1.45 (18.744)	13.33 (27.603)	-2.38 (27.625)	8.77 (20.036)	0.00 (22.222)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7

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Table GERMANY.2.5.4.3

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Appetite Loss

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	15	15	10	9	25	24
Mean (SD)	2.22 (8.607)	-4.44 (11.729)	16.67 (17.568)	-3.70 (11.111)	8.00 (14.530)	-4.17 (11.261)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 0.0
Week 88						
n	8	8	6	6	14	14
Mean (SD)	4.17 (11.785)	0.00 (0.000)	16.67 (18.257)	-5.56 (32.773)	9.52 (15.627)	-2.38 (20.524)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 0.0	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Appetite Loss

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	0.00 (0.000)	0.00 (0.000)	66.67 (0.000)	16.67 (23.570)	26.67 (36.515)	6.67 (14.907)
Median	0.00	0.00	66.67	16.67	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	0.0, 33.3	0.0, 66.7	0.0, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Constipation

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	41		18		59	
Mean (SD)	12.20 (24.445)		14.81 (20.523)		12.99 (23.174)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	40	40	20	18		
Mean (SD)	9.17 (21.334)	-3.33 (18.179)	21.67 (32.936)	7.41 (29.273)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

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Constipation

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	41	41	19	18		
Mean (SD)	9.76 (23.856)	-2.44 (21.590)	21.05 (31.838)	7.41 (26.948)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-33.3, 66.7		
Week 8						
n	32	32	14	13		
Mean (SD)	5.21 (14.930)	-6.25 (21.482)	19.05 (33.878)	7.69 (14.618)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	0.0, 33.3		

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Constipation

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	39	39	19	18		
Mean (SD)	9.40 (22.876)	-1.71 (20.158)	22.81 (35.229)	9.26 (25.063)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-33.3, 66.7		
Week 24						
n	36	36	18	17		
Mean (SD)	7.41 (19.698)	-2.78 (20.119)	16.67 (28.583)	1.96 (21.955)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

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Constipation

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	33	33	16	16	49	49
Mean (SD)	7.07 (13.838)	-7.07 (23.210)	12.50 (20.638)	-2.08 (14.751)	8.84 (16.352)	-5.44 (20.802)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-66.7, 33.3
Week 56						
n	23	23	15	14	38	37
Mean (SD)	7.25 (22.375)	-10.14 (21.165)	13.33 (21.082)	0.00 (13.074)	9.65 (21.793)	-6.31 (18.983)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 0.0	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-66.7, 33.3

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Constipation

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	15	15	10	9	25	24
Mean (SD)	13.33 (27.603)	-6.67 (22.537)	10.00 (16.102)	0.00 (16.667)	12.00 (23.333)	-4.17 (20.412)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 33.3
Week 88						
n	8	8	6	6	14	14
Mean (SD)	16.67 (17.817)	0.00 (25.198)	33.33 (51.640)	22.22 (50.185)	23.81 (35.635)	9.52 (37.958)
Median	16.67	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Constipation

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	11.11 (19.245)	0.00 (0.000)	33.33 (47.140)	0.00 (47.140)	20.00 (29.814)	0.00 (23.570)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 0.0	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Diarrhea

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	41		18		59	
Mean (SD)	7.32 (19.021)		16.67 (23.570)		10.17 (20.765)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	41	41	20	18		
Mean (SD)	8.13 (20.791)	0.81 (20.396)	5.00 (16.312)	-11.11 (22.866)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 100.0	0.0, 66.7	-66.7, 33.3		

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Table GERMANY.2.5.4.3
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Diarrhea

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	41	41	19	18		
Mean (SD)	6.50 (15.311)	-0.81 (22.959)	3.51 (10.510)	-12.96 (20.256)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-100.0, 66.7	0.0, 33.3	-66.7, 0.0		
Week 8						
n	32	32	14	13		
Mean (SD)	10.42 (17.835)	3.13 (22.969)	7.14 (19.298)	-12.82 (16.879)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 66.7	0.0, 66.7	-33.3, 0.0		

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Diarrhea

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	39	39	19	18		
Mean (SD)	5.13 (12.184)	-2.56 (16.013)	0.00 (0.000)	-16.67 (23.570)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 0.0	-66.7, 0.0		
Week 24						
n	36	36	18	17		
Mean (SD)	5.56 (12.599)	-0.93 (16.877)	5.56 (12.783)	-9.80 (19.596)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 0.0		

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Diarrhea

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	33	33	16	16	49	49
Mean (SD)	7.07 (13.838)	-2.02 (24.916)	6.25 (13.437)	-10.42 (23.472)	6.80 (13.574)	-4.76 (24.533)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-100.0, 33.3
Week 56						
n	23	23	15	14	38	37
Mean (SD)	10.14 (15.682)	2.90 (26.425)	8.89 (15.258)	-11.90 (24.832)	9.65 (15.320)	-2.70 (26.502)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-100.0, 33.3

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Table GERMANY.2.5.4.3
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Diarrhea
< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	14	14	10	9	24	23
Mean (SD)	0.00 (0.000)	-2.38 (8.909)	16.67 (23.570)	-7.41 (14.699)	6.94 (16.966)	-4.35 (11.478)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 66.7	-33.3, 0.0	0.0, 66.7	-33.3, 0.0
Week 88						
n	8	8	6	6	14	14
Mean (SD)	0.00 (0.000)	0.00 (0.000)	5.56 (13.608)	-16.67 (27.889)	2.38 (8.909)	-7.14 (19.298)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Diarrhea

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Financial Difficulties

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	41		18		59	
Mean (SD)	22.76 (32.006)		25.93 (33.442)		23.73 (32.194)	
Median	0.00		16.67		0.00	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	41	41	20	18		
Mean (SD)	21.95 (32.154)	-0.81 (15.790)	28.33 (36.314)	5.56 (30.785)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 100.0		

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Financial Difficulties

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	41	41	19	18		
Mean (SD)	19.51 (28.844)	-3.25 (22.117)	22.81 (36.939)	-1.85 (33.279)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 100.0		
Week 8						
n	32	32	14	13		
Mean (SD)	12.50 (23.570)	-8.33 (22.401)	16.67 (25.318)	2.56 (21.350)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 66.7	-33.3, 33.3		

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Modified Randomized Set

Financial Difficulties

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	39	39	19	18		
Mean (SD)	18.80 (28.403)	-3.42 (18.409)	28.07 (38.910)	3.70 (35.954)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 100.0		
Week 24						
n	36	36	18	17		
Mean (SD)	21.30 (32.025)	-0.93 (24.542)	25.93 (38.866)	0.00 (33.333)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 100.0	0.0, 100.0	-66.7, 100.0		

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Financial Difficulties

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	33	33	16	16	49	49
Mean (SD)	20.20 (29.976)	-5.05 (25.168)	20.83 (34.157)	-8.33 (39.441)	20.41 (31.044)	-6.12 (30.180)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-100.0, 100.0	0.0, 100.0	-100.0, 100.0
Week 56						
n	23	23	15	14	38	37
Mean (SD)	13.04 (24.077)	-10.14 (21.165)	26.67 (36.078)	-2.38 (42.294)	18.42 (29.711)	-7.21 (30.566)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 100.0	0.0, 100.0	-66.7, 100.0

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Table GERMANY.2.5.4.3

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Financial Difficulties

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	14	14	10	9	24	23
Mean (SD)	9.52 (15.627)	-11.90 (28.063)	10.00 (22.498)	-18.52 (24.216)	9.72 (18.334)	-14.49 (26.258)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-66.7, 0.0	0.0, 66.7	-66.7, 33.3
Week 88						
n	8	8	6	6	14	14
Mean (SD)	8.33 (15.430)	-16.67 (25.198)	5.56 (13.608)	-33.33 (29.814)	7.14 (14.194)	-23.81 (27.514)
Median	0.00	0.00	0.00	-33.33	0.00	-16.67
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Financial Difficulties

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	0.00 (0.000)	-22.22 (38.490)	16.67 (23.570)	-66.67 (0.000)	6.67 (14.907)	-40.00 (36.515)
Median	0.00	0.00	16.67	-66.67	0.00	-66.67
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 33.3	-66.7, -66.7	0.0, 33.3	-66.7, 0.0

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Fatigue

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean (SD)	34.03 (22.395)		35.19 (17.801)		34.34 (20.832)	
Median	33.33		27.78		33.33	
Min, Max	0.0, 88.9		22.2, 66.7		0.0, 88.9	
Week 2						
n	15	15	5	5		
Mean (SD)	33.33 (25.545)	0.00 (14.548)	40.00 (14.907)	11.11 (15.713)		
Median	33.33	0.00	33.33	11.11		
Min, Max	0.0, 100.0	-22.2, 22.2	33.3, 66.7	-11.1, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Fatigue
≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	16	16	6	6		
Mean (SD)	27.43 (20.833)	-6.60 (20.207)	48.15 (19.458)	12.96 (14.768)		
Median	33.33	0.00	38.89	11.11		
Min, Max	0.0, 77.8	-55.6, 22.2	33.3, 77.8	-11.1, 33.3		
Week 8						
n	13	13	5	5		
Mean (SD)	35.90 (26.510)	2.56 (19.852)	37.78 (23.040)	8.89 (24.088)		
Median	33.33	0.00	33.33	11.11		
Min, Max	0.0, 100.0	-22.2, 44.4	22.2, 77.8	-22.2, 44.4		

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Fatigue

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	13	13	6	6		
Mean (SD)	29.06 (16.690)	-5.98 (17.925)	48.15 (27.815)	12.96 (29.327)		
Median	33.33	0.00	38.89	5.56		
Min, Max	0.0, 55.6	-44.4, 22.2	22.2, 100.0	-11.1, 66.7		
Week 24						
n	13	13	6	6		
Mean (SD)	29.06 (21.052)	-5.98 (20.596)	38.89 (13.608)	3.70 (11.476)		
Median	33.33	-11.11	44.44	0.00		
Min, Max	0.0, 66.7	-44.4, 33.3	22.2, 55.6	-11.1, 22.2		

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Fatigue

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	13	13	5	5	18	18
Mean (SD)	26.50 (17.881)	-6.84 (16.690)	57.78 (38.809)	20.00 (30.832)	35.19 (28.070)	0.62 (23.944)
Median	22.22	0.00	33.33	11.11	27.78	0.00
Min, Max	0.0, 66.7	-44.4, 22.2	22.2, 100.0	-11.1, 66.7	0.0, 100.0	-44.4, 66.7
Week 56						
n	11	11	2	2	13	13
Mean (SD)	32.32 (21.917)	-1.01 (25.070)	33.33 (15.713)	-11.11 (15.713)	32.48 (20.519)	-2.56 (23.637)
Median	33.33	0.00	33.33	-11.11	33.33	0.00
Min, Max	0.0, 66.7	-44.4, 44.4	22.2, 44.4	-22.2, 0.0	0.0, 66.7	-44.4, 44.4

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Fatigue

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	6	6	2	2	8	8
Mean (SD)	25.93 (20.688)	-12.96 (10.924)	66.67 (47.140)	16.67 (70.711)	36.11 (31.286)	-5.56 (31.427)
Median	22.22	-11.11	66.67	16.67	27.78	-11.11
Min, Max	0.0, 55.6	-33.3, 0.0	33.3, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 66.7
Week 88						
n	6	6	2	2	8	8
Mean (SD)	24.07 (20.387)	-14.81 (27.815)	55.56 (31.427)	5.56 (54.997)	31.94 (25.502)	-9.72 (32.766)
Median	22.22	-22.22	55.56	5.56	33.33	-22.22
Min, Max	0.0, 55.6	-44.4, 33.3	33.3, 77.8	-33.3, 44.4	0.0, 77.8	-44.4, 44.4

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Fatigue

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	33.33 (NA)	-55.56 (NA)			33.33 (NA)	-55.56 (NA)
Median	33.33	-55.56			33.33	-55.56
Min, Max	33.3, 33.3	-55.6, -55.6			33.3, 33.3	-55.6, -55.6

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Modified Randomized Set

Nausea and Vomiting

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean (SD)	0.00 (0.000)		0.00 (0.000)		0.00 (0.000)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 0.0		0.0, 0.0		0.0, 0.0	
Week 2						
n	15	15	5	5		
Mean (SD)	3.33 (12.910)	3.33 (12.910)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 50.0	0.0, 50.0	0.0, 0.0	0.0, 0.0		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Nausea and Vomiting

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	16	16	6	6		
Mean (SD)	3.13 (9.065)	3.13 (9.065)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 0.0	0.0, 0.0		
Week 8						
n	13	13	5	5		
Mean (SD)	0.00 (0.000)	0.00 (0.000)	3.33 (7.454)	3.33 (7.454)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 16.7	0.0, 16.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Nausea and Vomiting

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	13	13	6	6		
Mean (SD)	2.56 (9.245)	2.56 (9.245)	2.78 (6.804)	2.78 (6.804)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 16.7	0.0, 16.7		
Week 24						
n	13	13	6	6		
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0		

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Nausea and Vomiting

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	13	13	5	5	18	18
Mean (SD)	1.28 (4.623)	1.28 (4.623)	10.00 (22.361)	10.00 (22.361)	3.70 (12.201)	3.70 (12.201)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	0.0, 16.7	0.0, 50.0	0.0, 50.0	0.0, 50.0	0.0, 50.0
Week 56						
n	11	11	2	2	13	13
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0

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Nausea and Vomiting

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	6	6	2	2	8	8
Mean (SD)	0.00 (0.000)	0.00 (0.000)	25.00 (11.785)	25.00 (11.785)	6.25 (12.400)	6.25 (12.400)
Median	0.00	0.00	25.00	25.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	16.7, 33.3	16.7, 33.3	0.0, 33.3	0.0, 33.3
Week 88						
n	6	6	2	2	8	8
Mean (SD)	0.00 (0.000)	0.00 (0.000)	8.33 (11.785)	8.33 (11.785)	2.08 (5.893)	2.08 (5.893)
Median	0.00	0.00	8.33	8.33	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 16.7	0.0, 16.7	0.0, 16.7	0.0, 16.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Nausea and Vomiting

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	0.00 (NA)			0.00 (NA)	0.00 (NA)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Pain

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean (SD)	11.46 (25.617)		13.89 (16.387)		12.12 (23.107)	
Median	0.00		8.33		0.00	
Min, Max	0.0, 100.0		0.0, 33.3		0.0, 100.0	
Week 2						
n	15	15	4	4		
Mean (SD)	15.56 (29.859)	3.33 (22.003)	20.83 (8.333)	4.17 (15.957)		
Median	0.00	0.00	16.67	8.33		
Min, Max	0.0, 100.0	-33.3, 66.7	16.7, 33.3	-16.7, 16.7		

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Modified Randomized Set

Pain

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	16	16	6	6		
Mean (SD)	13.54 (27.365)	2.08 (13.437)	19.44 (16.387)	5.56 (8.607)		
Median	0.00	0.00	25.00	0.00		
Min, Max	0.0, 100.0	-16.7, 50.0	0.0, 33.3	0.0, 16.7		
Week 8						
n	12	12	4	4		
Mean (SD)	5.56 (14.794)	-1.39 (19.408)	12.50 (15.957)	-4.17 (20.972)		
Median	0.00	0.00	8.33	0.00		
Min, Max	0.0, 50.0	-33.3, 50.0	0.0, 33.3	-33.3, 16.7		

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Modified Randomized Set

Pain

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	13	13	5	5		
Mean (SD)	8.97 (27.735)	-3.85 (12.084)	23.33 (14.907)	10.00 (22.361)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 16.7	0.0, 33.3	-16.7, 33.3		
Week 24						
n	13	13	6	6		
Mean (SD)	14.10 (28.744)	1.28 (12.659)	11.11 (13.608)	-2.78 (16.387)		
Median	0.00	0.00	8.33	0.00		
Min, Max	0.0, 100.0	-16.7, 33.3	0.0, 33.3	-33.3, 16.7		

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Pain

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	13	13	5	5	18	18
Mean (SD)	15.38 (25.875)	1.28 (15.901)	50.00 (47.140)	40.00 (50.827)	25.00 (35.355)	12.04 (33.238)
Median	0.00	0.00	33.33	33.33	8.33	0.00
Min, Max	0.0, 83.3	-16.7, 33.3	0.0, 100.0	-16.7, 100.0	0.0, 100.0	-16.7, 100.0
Week 56						
n	11	11	2	2	13	13
Mean (SD)	24.24 (34.451)	7.58 (22.808)	33.33 (47.140)	16.67 (70.711)	25.64 (34.437)	8.97 (29.357)
Median	0.00	0.00	33.33	16.67	0.00	0.00
Min, Max	0.0, 100.0	-16.7, 66.7	0.0, 66.7	-33.3, 66.7	0.0, 100.0	-33.3, 66.7

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Table GERMANY.2.5.4.3
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Pain
≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	6	6	2	2	8	8
Mean (SD)	16.67 (25.820)	-2.78 (19.484)	66.67 (47.140)	58.33 (35.355)	29.17 (36.460)	12.50 (35.355)
Median	8.33	0.00	66.67	58.33	16.67	8.33
Min, Max	0.0, 66.7	-33.3, 16.7	33.3, 100.0	33.3, 83.3	0.0, 100.0	-33.3, 83.3
Week 88						
n	6	6	2	2	8	8
Mean (SD)	13.89 (26.701)	-5.56 (17.213)	66.67 (23.570)	58.33 (11.785)	27.08 (34.431)	10.42 (33.259)
Median	0.00	0.00	66.67	58.33	8.33	0.00
Min, Max	0.0, 66.7	-33.3, 16.7	50.0, 83.3	50.0, 66.7	0.0, 83.3	-33.3, 66.7

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Pain

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	33.33 (NA)	-66.67 (NA)			33.33 (NA)	-66.67 (NA)
Median	33.33	-66.67			33.33	-66.67
Min, Max	33.3, 33.3	-66.7, -66.7			33.3, 33.3	-66.7, -66.7

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Dyspnoea

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean (SD)	39.58 (21.837)		27.78 (25.092)		36.36 (22.792)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 66.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	16	16	5	5		
Mean (SD)	27.08 (25.000)	-12.50 (20.638)	46.67 (18.257)	13.33 (18.257)		
Median	33.33	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	33.3, 66.7	0.0, 33.3		

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Dyspnoea

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	16	16	6	6		
Mean (SD)	20.83 (26.874)	-18.75 (24.248)	38.89 (25.092)	11.11 (17.213)		
Median	16.67	-33.33	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	0.0, 33.3		
Week 8						
n	13	13	5	5		
Mean (SD)	30.77 (28.744)	-10.26 (21.014)	40.00 (14.907)	6.67 (27.889)		
Median	33.33	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	33.3, 66.7	-33.3, 33.3		

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Dyspnoea

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	13	13	6	6		
Mean (SD)	20.51 (21.681)	-17.95 (22.008)	33.33 (21.082)	5.56 (25.092)		
Median	33.33	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 66.7	-33.3, 33.3		
Week 24						
n	13	13	6	6		
Mean (SD)	25.64 (24.167)	-12.82 (21.681)	22.22 (17.213)	-5.56 (13.608)		
Median	33.33	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 33.3	-33.3, 0.0		

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Dyspnoea

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	13	13	5	5	18	18
Mean (SD)	20.51 (21.681)	-17.95 (22.008)	13.33 (18.257)	-13.33 (18.257)	18.52 (20.523)	-16.67 (20.612)
Median	33.33	0.00	0.00	0.00	16.67	0.00
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-66.7, 0.0
Week 56						
n	11	11	2	2	13	13
Mean (SD)	27.27 (32.722)	-9.09 (30.151)	16.67 (23.570)	16.67 (23.570)	25.64 (30.894)	-5.13 (29.957)
Median	33.33	0.00	16.67	16.67	33.33	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	0.0, 33.3	0.0, 100.0	-66.7, 33.3

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Dyspnoea

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	6	6	2	2	8	8
Mean (SD)	16.67 (27.889)	-22.22 (27.217)	33.33 (47.140)	16.67 (23.570)	20.83 (30.538)	-12.50 (30.538)
Median	0.00	-16.67	33.33	16.67	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 66.7	0.0, 33.3	0.0, 66.7	-66.7, 33.3
Week 88						
n	6	6	2	2	8	8
Mean (SD)	5.56 (13.608)	-33.33 (21.082)	0.00 (0.000)	-16.67 (23.570)	4.17 (11.785)	-29.17 (21.362)
Median	0.00	-33.33	0.00	-16.67	0.00	-33.33
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 0.0	-33.3, 0.0	0.0, 33.3	-66.7, 0.0

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Dyspnoea

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	-66.67 (NA)			0.00 (NA)	-66.67 (NA)
Median	0.00	-66.67			0.00	-66.67
Min, Max	0.0, 0.0	-66.7, -66.7			0.0, 0.0	-66.7, -66.7

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Insomnia

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean (SD)	18.75 (24.248)		5.56 (13.608)		15.15 (22.366)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 33.3		0.0, 66.7	
Week 2						
n	15	15	5	5		
Mean (SD)	26.67 (31.371)	6.67 (18.687)	13.33 (18.257)	13.33 (18.257)		
Median	33.33	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 33.3	0.0, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Insomnia

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	16	16	6	6		
Mean (SD)	16.67 (27.217)	-2.08 (22.669)	5.56 (13.608)	0.00 (21.082)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 33.3	-33.3, 33.3		
Week 8						
n	13	13	5	5		
Mean (SD)	20.51 (25.598)	5.13 (18.490)	13.33 (18.257)	13.33 (18.257)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 33.3	0.0, 33.3		

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Insomnia

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	13	13	6	6		
Mean (SD)	15.38 (22.008)	-2.56 (21.350)	11.11 (17.213)	5.56 (25.092)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 33.3	-33.3, 33.3		
Week 24						
n	13	13	6	6		
Mean (SD)	15.38 (22.008)	-2.56 (34.592)	5.56 (13.608)	0.00 (21.082)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 66.7	0.0, 33.3	-33.3, 33.3		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Insomnia

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	13	13	5	5	18	18
Mean (SD)	20.51 (21.681)	2.56 (25.318)	26.67 (43.461)	20.00 (29.814)	22.22 (28.006)	7.41 (26.948)
Median	33.33	0.00	0.00	0.00	16.67	0.00
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 100.0	0.0, 66.7	0.0, 100.0	-33.3, 66.7
Week 56						
n	11	11	2	2	13	13
Mean (SD)	24.24 (21.556)	6.06 (32.722)	50.00 (23.570)	33.33 (0.000)	28.21 (22.958)	10.26 (31.578)
Median	33.33	0.00	50.00	33.33	33.33	0.00
Min, Max	0.0, 66.7	-33.3, 66.7	33.3, 66.7	33.3, 33.3	0.0, 66.7	-33.3, 66.7

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Insomnia

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	6	6	2	2	8	8
Mean (SD)	11.11 (17.213)	-11.11 (17.213)	66.67 (0.000)	50.00 (23.570)	25.00 (29.547)	4.17 (33.034)
Median	0.00	0.00	66.67	50.00	16.67	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	66.7, 66.7	33.3, 66.7	0.0, 66.7	-33.3, 66.7
Week 88						
n	6	6	2	2	8	8
Mean (SD)	5.56 (13.608)	-16.67 (18.257)	16.67 (23.570)	0.00 (0.000)	8.33 (15.430)	-12.50 (17.252)
Median	0.00	-16.67	16.67	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	0.0, 0.0	0.0, 33.3	-33.3, 0.0

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Table GERMANY.2.5.4.3
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Insomnia

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	-66.67 (NA)			0.00 (NA)	-66.67 (NA)
Median	0.00	-66.67			0.00	-66.67
Min, Max	0.0, 0.0	-66.7, -66.7			0.0, 0.0	-66.7, -66.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Appetite Loss

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean (SD)	12.50 (20.638)		5.56 (13.608)		10.61 (18.930)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 33.3		0.0, 66.7	
Week 2						
n	16	16	5	5		
Mean (SD)	16.67 (24.343)	4.17 (20.638)	13.33 (18.257)	6.67 (14.907)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 33.3	0.0, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Appetite Loss

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	16	16	6	6		
Mean (SD)	8.33 (19.245)	-4.17 (11.386)	5.56 (13.608)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 33.3	0.0, 0.0		
Week 8						
n	13	13	5	5		
Mean (SD)	23.08 (25.036)	7.69 (27.735)	13.33 (18.257)	6.67 (14.907)		
Median	33.33	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 33.3	0.0, 33.3		

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Appetite Loss

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	13	13	6	6		
Mean (SD)	12.82 (21.681)	0.00 (30.429)	16.67 (27.889)	11.11 (17.213)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	0.0, 33.3		
Week 24						
n	13	13	6	6		
Mean (SD)	12.82 (16.879)	0.00 (30.429)	16.67 (27.889)	11.11 (17.213)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	0.0, 33.3		

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Table GERMANY.2.5.4.3

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Modified Randomized Set

Appetite Loss

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	13	13	5	5	18	18
Mean (SD)	15.38 (17.296)	5.13 (22.958)	26.67 (43.461)	20.00 (44.721)	18.52 (26.127)	9.26 (29.826)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	0.0, 100.0	0.0, 100.0	-33.3, 100.0
Week 56						
n	11	11	2	2	13	13
Mean (SD)	12.12 (16.817)	3.03 (23.355)	0.00 (0.000)	0.00 (0.000)	10.26 (16.013)	2.56 (21.350)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 0.0	0.0, 0.0	0.0, 33.3	-33.3, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Appetite Loss

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	6	6	2	2	8	8
Mean (SD)	5.56 (13.608)	-5.56 (13.608)	33.33 (47.140)	33.33 (47.140)	12.50 (24.801)	4.17 (27.817)
Median	0.00	0.00	33.33	33.33	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 66.7	0.0, 66.7	0.0, 66.7	-33.3, 66.7
Week 88						
n	6	6	2	2	8	8
Mean (SD)	11.11 (17.213)	0.00 (21.082)	16.67 (23.570)	16.67 (23.570)	12.50 (17.252)	4.17 (21.362)
Median	0.00	0.00	16.67	16.67	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3	-33.3, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Appetite Loss

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	0.00 (NA)			0.00 (NA)	0.00 (NA)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Constipation

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean (SD)	16.67 (21.082)		0.00 (0.000)		12.12 (19.370)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 0.0		0.0, 66.7	
Week 2						
n	15	15	5	5		
Mean (SD)	11.11 (27.217)	-6.67 (22.537)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 0.0	0.0, 0.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Constipation

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	16	16	6	6		
Mean (SD)	10.42 (20.069)	-6.25 (21.837)	11.11 (17.213)	11.11 (17.213)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 33.3	0.0, 33.3		
Week 8						
n	13	13	5	5		
Mean (SD)	7.69 (14.618)	-7.69 (19.971)	6.67 (14.907)	6.67 (14.907)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	0.0, 33.3		

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Modified Randomized Set

Constipation

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	13	13	6	6		
Mean (SD)	10.26 (28.495)	-10.26 (21.014)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 0.0	0.0, 0.0		
Week 24						
n	13	13	6	6		
Mean (SD)	5.13 (18.490)	-15.38 (17.296)	16.67 (18.257)	16.67 (18.257)		
Median	0.00	0.00	16.67	16.67		
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 33.3	0.0, 33.3		

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Constipation

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	13	13	5	5	18	18
Mean (SD)	5.13 (18.490)	-12.82 (16.879)	26.67 (27.889)	26.67 (27.889)	11.11 (22.866)	-1.85 (26.746)
Median	0.00	0.00	33.33	33.33	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 66.7	0.0, 66.7	0.0, 66.7	-33.3, 66.7
Week 56						
n	11	11	2	2	13	13
Mean (SD)	15.15 (22.918)	-3.03 (17.979)	0.00 (0.000)	0.00 (0.000)	12.82 (21.681)	-2.56 (16.452)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 0.0	0.0, 0.0	0.0, 66.7	-33.3, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Constipation

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	6	6	2	2	8	8
Mean (SD)	11.11 (17.213)	-11.11 (17.213)	16.67 (23.570)	16.67 (23.570)	12.50 (17.252)	-4.17 (21.362)
Median	0.00	0.00	16.67	16.67	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	0.0, 33.3	0.0, 33.3	-33.3, 33.3
Week 88						
n	6	6	2	2	8	8
Mean (SD)	11.11 (17.213)	-11.11 (17.213)	0.00 (0.000)	0.00 (0.000)	8.33 (15.430)	-8.33 (15.430)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 0.0	0.0, 0.0	0.0, 33.3	-33.3, 0.0

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Constipation

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	33.33 (NA)	-33.33 (NA)			33.33 (NA)	-33.33 (NA)
Median	33.33	-33.33			33.33	-33.33
Min, Max	33.3, 33.3	-33.3, -33.3			33.3, 33.3	-33.3, -33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Diarrhea
≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean (SD)	0.00 (0.000)		0.00 (0.000)		0.00 (0.000)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 0.0		0.0, 0.0		0.0, 0.0	
Week 2						
n	15	15	5	5		
Mean (SD)	0.00 (0.000)	0.00 (0.000)	6.67 (14.907)	6.67 (14.907)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 33.3	0.0, 33.3		

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Modified Randomized Set

Diarrhea

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	16	16	6	6		
Mean (SD)	2.08 (8.333)	2.08 (8.333)	5.56 (13.608)	5.56 (13.608)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3		
Week 8						
n	13	13	5	5		
Mean (SD)	0.00 (0.000)	0.00 (0.000)	6.67 (14.907)	6.67 (14.907)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 33.3	0.0, 33.3		

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Modified Randomized Set

Diarrhea

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	13	13	6	6		
Mean (SD)	7.69 (14.618)	7.69 (14.618)	16.67 (27.889)	16.67 (27.889)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 66.7	0.0, 66.7		
Week 24						
n	13	13	6	6		
Mean (SD)	5.13 (12.518)	5.13 (12.518)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 0.0	0.0, 0.0		

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Modified Randomized Set

Diarrhea

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	13	13	5	5	18	18
Mean (SD)	0.00 (0.000)	0.00 (0.000)	13.33 (18.257)	13.33 (18.257)	3.70 (10.779)	3.70 (10.779)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3
Week 56						
n	11	11	2	2	13	13
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Diarrhea

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	6	6	2	2	8	8
Mean (SD)	0.00 (0.000)	0.00 (0.000)	66.67 (47.140)	66.67 (47.140)	16.67 (35.635)	16.67 (35.635)
Median	0.00	0.00	66.67	66.67	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	33.3, 100.0	33.3, 100.0	0.0, 100.0	0.0, 100.0
Week 88						
n	6	6	2	2	8	8
Mean (SD)	5.56 (13.608)	5.56 (13.608)	16.67 (23.570)	16.67 (23.570)	8.33 (15.430)	8.33 (15.430)
Median	0.00	0.00	16.67	16.67	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3

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Table GERMANY.2.5.4.3

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Diarrhea

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	0.00 (NA)			0.00 (NA)	0.00 (NA)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Financial Difficulties

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean (SD)	14.58 (27.131)		16.67 (27.889)		15.15 (26.681)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	16	16	5	5		
Mean (SD)	8.33 (19.245)	-6.25 (13.437)	6.67 (14.907)	-6.67 (14.907)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 33.3	-33.3, 0.0		

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Financial Difficulties

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	16	16	6	6		
Mean (SD)	12.50 (20.638)	-2.08 (14.751)	5.56 (13.608)	-11.11 (17.213)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 33.3	-33.3, 0.0		
Week 8						
n	13	13	5	5		
Mean (SD)	12.82 (16.879)	-2.56 (21.350)	6.67 (14.907)	-6.67 (14.907)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-33.3, 0.0		

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Financial Difficulties

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	13	13	6	6		
Mean (SD)	15.38 (22.008)	0.00 (13.608)	5.56 (13.608)	-11.11 (17.213)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 33.3	-33.3, 0.0		
Week 24						
n	13	13	6	6		
Mean (SD)	7.69 (14.618)	-7.69 (27.735)	11.11 (17.213)	-5.56 (13.608)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-100.0, 0.0	0.0, 33.3	-33.3, 0.0		

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Financial Difficulties

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	13	13	5	5	18	18
Mean (SD)	10.26 (16.013)	-5.13 (18.490)	20.00 (29.814)	0.00 (23.570)	12.96 (20.256)	-3.70 (19.433)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-66.7, 33.3
Week 56						
n	11	11	2	2	13	13
Mean (SD)	12.12 (22.473)	-6.06 (13.484)	16.67 (23.570)	0.00 (0.000)	12.82 (21.681)	-5.13 (12.518)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 33.3	0.0, 0.0	0.0, 66.7	-33.3, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Financial Difficulties

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	6	6	2	2	8	8
Mean (SD)	5.56 (13.608)	-5.56 (13.608)	16.67 (23.570)	0.00 (0.000)	8.33 (15.430)	-4.17 (11.785)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	0.0, 0.0	0.0, 33.3	-33.3, 0.0
Week 88						
n	6	6	2	2	8	8
Mean (SD)	11.11 (17.213)	0.00 (0.000)	16.67 (23.570)	0.00 (0.000)	12.50 (17.252)	0.00 (0.000)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 0.0	0.0, 33.3	0.0, 0.0	0.0, 33.3	0.0, 0.0

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Table GERMANY.2.5.4.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Financial Difficulties

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	0.00 (NA)			0.00 (NA)	0.00 (NA)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Fatigue

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	25		12		37	
Mean (SD)	44.00 (24.737)		47.22 (24.216)		45.05 (24.279)	
Median	44.44		38.89		44.44	
Min, Max	0.0, 88.9		11.1, 100.0		0.0, 100.0	
Week 2						
n	24	24	13	12		
Mean (SD)	34.72 (30.616)	-9.26 (23.551)	48.72 (22.923)	4.63 (18.015)		
Median	33.33	-11.11	44.44	0.00		
Min, Max	0.0, 100.0	-55.6, 33.3	11.1, 100.0	-22.2, 44.4		

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Fatigue

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	25	25	13	12		
Mean (SD)	29.11 (26.317)	-14.89 (25.495)	47.01 (24.069)	2.78 (23.271)		
Median	33.33	-22.22	33.33	0.00		
Min, Max	0.0, 100.0	-55.6, 44.4	11.1, 100.0	-44.4, 44.4		
Week 8						
n	19	19	8	7		
Mean (SD)	25.15 (24.242)	-18.13 (25.448)	44.44 (21.414)	4.76 (23.002)		
Median	22.22	-22.22	38.89	0.00		
Min, Max	0.0, 100.0	-55.6, 44.4	11.1, 77.8	-33.3, 44.4		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Fatigue

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	23	23	13	12		
Mean (SD)	29.47 (18.835)	-15.94 (18.296)	57.26 (34.500)	8.33 (34.532)		
Median	33.33	-22.22	55.56	0.00		
Min, Max	0.0, 66.7	-44.4, 22.2	0.0, 100.0	-44.4, 77.8		
Week 24						
n	20	20	13	12		
Mean (SD)	30.56 (30.562)	-14.44 (26.269)	37.61 (31.927)	-9.26 (39.308)		
Median	22.22	-22.22	33.33	-16.67		
Min, Max	0.0, 100.0	-55.6, 33.3	0.0, 100.0	-55.6, 77.8		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Fatigue

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	12	12	30	30
Mean (SD)	33.33 (30.489)	-12.96 (19.526)	42.59 (31.367)	-4.63 (36.224)	37.04 (30.650)	-9.63 (27.175)
Median	22.22	-11.11	33.33	-5.56	33.33	-11.11
Min, Max	0.0, 100.0	-44.4, 33.3	0.0, 100.0	-55.6, 66.7	0.0, 100.0	-55.6, 66.7
Week 56						
n	12	12	11	10	23	22
Mean (SD)	34.26 (29.761)	-12.04 (27.405)	34.34 (14.448)	-14.44 (30.564)	34.30 (23.189)	-13.13 (28.200)
Median	27.78	-22.22	33.33	-11.11	33.33	-11.11
Min, Max	0.0, 100.0	-44.4, 44.4	22.2, 66.7	-66.7, 44.4	0.0, 100.0	-66.7, 44.4

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Fatigue

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	8	7	16	15
Mean (SD)	30.56 (22.023)	-15.28 (13.197)	51.39 (25.845)	4.76 (32.620)	40.97 (25.570)	-5.93 (25.499)
Median	33.33	-11.11	44.44	0.00	33.33	-11.11
Min, Max	0.0, 55.6	-33.3, 0.0	22.2, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 66.7
Week 88						
n	6	6	5	5	11	11
Mean (SD)	18.52 (21.849)	-25.93 (29.537)	48.89 (18.592)	-6.67 (41.276)	32.32 (25.070)	-17.17 (34.912)
Median	11.11	-33.33	44.44	0.00	33.33	-33.33
Min, Max	0.0, 55.6	-44.4, 33.3	33.3, 77.8	-66.7, 44.4	0.0, 77.8	-66.7, 44.4

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Fatigue

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	2	2	3	3
Mean (SD)	33.33 (NA)	-55.56 (NA)	27.78 (7.857)	-38.89 (54.997)	29.63 (6.415)	-44.44 (40.062)
Median	33.33	-55.56	27.78	-38.89	33.33	-55.56
Min, Max	33.3, 33.3	-55.6, -55.6	22.2, 33.3	-77.8, 0.0	22.2, 33.3	-77.8, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Nausea and Vomiting

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	25		12		37	
Mean (SD)	0.67 (3.333)		4.17 (10.360)		1.80 (6.554)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 16.7		0.0, 33.3		0.0, 33.3	
Week 2						
n	24	24	13	12		
Mean (SD)	6.25 (14.590)	5.56 (14.469)	6.41 (12.799)	2.78 (6.487)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 50.0	0.0, 50.0	0.0, 33.3	0.0, 16.7		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
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Nausea and Vomiting

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	25	25	13	12		
Mean (SD)	6.67 (12.729)	6.00 (11.667)	2.56 (6.259)	-1.39 (11.143)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 16.7	-33.3, 16.7		
Week 8						
n	19	19	8	7		
Mean (SD)	0.88 (3.824)	0.00 (0.000)	6.25 (8.626)	7.14 (8.909)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 16.7	0.0, 0.0	0.0, 16.7	0.0, 16.7		

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Nausea and Vomiting

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	23	23	13	12		
Mean (SD)	2.90 (8.184)	2.17 (7.628)	14.10 (25.318)	6.94 (16.603)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 83.3	-16.7, 50.0		
Week 24						
n	20	20	13	12		
Mean (SD)	5.00 (16.312)	4.17 (16.992)	5.13 (8.006)	1.39 (15.006)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-16.7, 66.7	0.0, 16.7	-33.3, 16.7		

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Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	12	12	30	30
Mean (SD)	7.41 (11.747)	7.41 (11.747)	6.94 (15.006)	2.78 (17.164)	7.22 (12.898)	5.56 (14.070)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 50.0	-16.7, 50.0	0.0, 50.0	-16.7, 50.0
Week 56						
n	12	12	11	10	23	22
Mean (SD)	0.00 (0.000)	0.00 (0.000)	13.64 (29.644)	10.00 (29.606)	6.52 (21.165)	4.55 (20.041)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 100.0	-33.3, 83.3	0.0, 100.0	-33.3, 83.3

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Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	8	7	16	15
Mean (SD)	0.00 (0.000)	0.00 (0.000)	8.33 (12.599)	4.76 (20.893)	4.17 (9.623)	2.22 (13.897)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3
Week 88						
n	6	6	5	5	11	11
Mean (SD)	0.00 (0.000)	0.00 (0.000)	6.67 (9.129)	0.00 (20.412)	3.03 (6.742)	0.00 (12.910)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 16.7	-33.3, 16.7	0.0, 16.7	-33.3, 16.7

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Nausea and Vomiting

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	2	2	3	3
Mean (SD)	0.00 (NA)	0.00 (NA)	50.00 (23.570)	33.33 (0.000)	33.33 (33.333)	22.22 (19.245)
Median	0.00	0.00	50.00	33.33	33.33	33.33
Min, Max	0.0, 0.0	0.0, 0.0	33.3, 66.7	33.3, 33.3	0.0, 66.7	0.0, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Pain

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	25		12		37	
Mean (SD)	15.33 (28.431)		22.22 (29.588)		17.57 (28.584)	
Median	0.00		16.67		0.00	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	24	24	12	11		
Mean (SD)	13.89 (28.937)	-2.08 (17.932)	25.00 (23.028)	3.03 (23.355)		
Median	0.00	0.00	25.00	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 66.7	-33.3, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Pain

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	25	25	13	12		
Mean (SD)	15.33 (27.605)	0.00 (10.758)	25.64 (33.065)	5.56 (28.721)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-16.7, 33.3	0.0, 100.0	-33.3, 83.3		
Week 8						
n	18	18	7	6		
Mean (SD)	3.70 (12.201)	-5.56 (9.901)	7.14 (8.909)	-8.33 (13.944)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 50.0	-33.3, 0.0	0.0, 16.7	-33.3, 0.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Pain

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	23	23	12	11		
Mean (SD)	13.04 (26.570)	-3.62 (15.039)	29.17 (38.353)	6.06 (39.632)		
Median	0.00	0.00	8.33	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 83.3		
Week 24						
n	20	20	13	12		
Mean (SD)	15.00 (31.016)	-2.50 (23.116)	11.54 (17.193)	-9.72 (25.084)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-50.0, 66.7	0.0, 50.0	-66.7, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Pain

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	12	12	30	30
Mean (SD)	17.59 (30.504)	0.93 (14.543)	29.17 (35.622)	6.94 (29.694)	22.22 (32.558)	3.33 (21.620)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 83.3	-33.3, 33.3	0.0, 100.0	-33.3, 83.3	0.0, 100.0	-33.3, 83.3
Week 56						
n	12	12	11	10	23	22
Mean (SD)	22.22 (35.056)	0.00 (15.891)	25.76 (20.226)	1.67 (19.954)	23.91 (28.349)	0.76 (17.425)
Median	0.00	0.00	16.67	0.00	16.67	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-33.3, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Pain

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	8	7	16	15
Mean (SD)	8.33 (23.570)	-8.33 (15.430)	29.17 (34.215)	14.29 (37.796)	18.75 (30.353)	2.22 (29.457)
Median	0.00	0.00	25.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 100.0	-16.7, 83.3	0.0, 100.0	-33.3, 83.3
Week 88						
n	6	6	5	5	11	11
Mean (SD)	11.11 (27.217)	-5.56 (13.608)	30.00 (32.059)	23.33 (27.889)	19.70 (29.644)	7.58 (25.126)
Median	0.00	0.00	16.67	16.67	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 83.3	0.0, 66.7	0.0, 83.3	-33.3, 66.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Pain

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	2	2	3	3
Mean (SD)	33.33 (NA)	-66.67 (NA)	16.67 (0.000)	8.33 (11.785)	22.22 (9.623)	-16.67 (44.096)
Median	33.33	-66.67	16.67	8.33	16.67	0.00
Min, Max	33.3, 33.3	-66.7, -66.7	16.7, 16.7	0.0, 16.7	16.7, 33.3	-66.7, 16.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Dyspnoea

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	25		12		37	
Mean (SD)	36.00 (30.307)		27.78 (23.925)		33.33 (28.328)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	25	25	13	12		
Mean (SD)	21.33 (27.012)	-14.67 (21.688)	33.33 (23.570)	8.33 (28.868)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	-33.3, 66.7		

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Modified Randomized Set

Dyspnoea

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	25	25	13	12		
Mean (SD)	13.33 (21.517)	-22.67 (24.944)	30.77 (37.172)	5.56 (34.329)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		
Week 8						
n	19	19	8	7		
Mean (SD)	19.30 (23.083)	-14.04 (20.233)	37.50 (33.034)	9.52 (31.706)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-33.3, 66.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Dyspnoea

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	23	23	13	12		
Mean (SD)	18.84 (22.079)	-17.39 (28.194)	38.46 (22.958)	11.11 (32.824)		
Median	0.00	0.00	33.33	16.67		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-33.3, 66.7		
Week 24						
n	20	20	13	12		
Mean (SD)	18.33 (25.305)	-15.00 (29.568)	17.95 (17.296)	-8.33 (20.719)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 33.3	-33.3, 33.3		

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Dyspnoea

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	12	12	30	30
Mean (SD)	25.93 (26.948)	-14.81 (28.520)	19.44 (17.164)	-8.33 (20.719)	23.33 (23.407)	-12.22 (25.496)
Median	33.33	0.00	33.33	0.00	33.33	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-66.7, 33.3
Week 56						
n	12	12	11	10	23	22
Mean (SD)	33.33 (34.816)	-8.33 (32.177)	12.12 (16.817)	-10.00 (27.442)	23.19 (29.189)	-9.09 (29.424)
Median	33.33	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 100.0	-66.7, 33.3

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Dyspnoea

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	8	7	16	15
Mean (SD)	25.00 (29.547)	-16.67 (17.817)	29.17 (27.817)	0.00 (33.333)	27.08 (27.806)	-8.89 (26.627)
Median	16.67	-16.67	33.33	0.00	33.33	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3
Week 88						
n	6	6	5	5	11	11
Mean (SD)	0.00 (0.000)	-27.78 (25.092)	13.33 (18.257)	-13.33 (29.814)	6.06 (13.484)	-21.21 (26.968)
Median	0.00	-33.33	0.00	-33.33	0.00	-33.33
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-66.7, 33.3

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Dyspnoea

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	2	2	3	3
Mean (SD)	0.00 (NA)	-66.67 (NA)	16.67 (23.570)	0.00 (0.000)	11.11 (19.245)	-22.22 (38.490)
Median	0.00	-66.67	16.67	0.00	0.00	0.00
Min, Max	0.0, 0.0	-66.7, -66.7	0.0, 33.3	0.0, 0.0	0.0, 33.3	-66.7, 0.0

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Insomnia

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	25		12		37	
Mean (SD)	28.00 (26.667)		33.33 (37.605)		29.73 (30.209)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 66.7		0.0, 100.0		0.0, 100.0	
Week 2						
n	24	24	13	12		
Mean (SD)	22.22 (32.103)	-6.94 (21.934)	33.33 (36.004)	0.00 (37.605)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 66.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Insomnia

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	25	25	13	12		
Mean (SD)	26.67 (33.333)	-1.33 (26.317)	33.33 (30.429)	0.00 (44.947)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 100.0	-100.0, 66.7		
Week 8						
n	19	19	8	7		
Mean (SD)	17.54 (23.223)	-8.77 (26.856)	4.17 (11.785)	-19.05 (37.796)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 33.3	-100.0, 0.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Insomnia

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	23	23	13	12		
Mean (SD)	18.84 (24.259)	-8.70 (18.027)	35.90 (41.859)	2.78 (43.712)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-66.7, 66.7		
Week 24						
n	20	20	13	12		
Mean (SD)	15.00 (27.519)	-10.00 (26.710)	23.08 (25.036)	-11.11 (25.950)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	-66.7, 33.3		

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Modified Randomized Set

Insomnia

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	12	12	30	30
Mean (SD)	27.78 (30.785)	-3.70 (25.280)	22.22 (32.824)	-11.11 (43.423)	25.56 (31.175)	-6.67 (33.218)
Median	33.33	0.00	0.00	0.00	16.67	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-100.0, 66.7	0.0, 100.0	-100.0, 66.7
Week 56						
n	12	12	11	10	23	22
Mean (SD)	22.22 (29.588)	-11.11 (29.588)	33.33 (33.333)	-3.33 (36.683)	27.54 (31.224)	-7.58 (32.419)
Median	16.67	-33.33	33.33	0.00	33.33	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Insomnia

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	8	7	16	15
Mean (SD)	12.50 (17.252)	-16.67 (17.817)	33.33 (30.861)	-4.76 (52.453)	22.92 (26.440)	-11.11 (37.090)
Median	0.00	-16.67	33.33	0.00	16.67	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-100.0, 66.7	0.0, 66.7	-100.0, 66.7
Week 88						
n	6	6	5	5	11	11
Mean (SD)	5.56 (13.608)	-22.22 (17.213)	40.00 (43.461)	6.67 (49.441)	21.21 (34.230)	-9.09 (36.790)
Median	0.00	-33.33	33.33	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Insomnia

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	2	2	3	3
Mean (SD)	0.00 (NA)	-66.67 (NA)	50.00 (70.711)	-16.67 (117.851)	33.33 (57.735)	-33.33 (88.192)
Median	0.00	-66.67	50.00	-16.67	0.00	-66.67
Min, Max	0.0, 0.0	-66.7, -66.7	0.0, 100.0	-100.0, 66.7	0.0, 100.0	-100.0, 66.7

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Appetite Loss

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	25		12		37	
Mean (SD)	6.67 (13.608)		13.89 (22.285)		9.01 (16.940)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 33.3		0.0, 66.7		0.0, 66.7	
Week 2						
n	25	25	13	12		
Mean (SD)	12.00 (21.257)	5.33 (20.817)	17.95 (25.875)	5.56 (12.975)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	0.0, 33.3		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties. Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem. Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan. D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point. Baseline is defined as the last non-missing value prior to the first dose of study drug. Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Appetite Loss

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	25	25	13	12		
Mean (SD)	4.00 (11.055)	-2.67 (13.333)	10.26 (28.495)	-2.78 (30.011)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-66.7, 66.7		
Week 8						
n	19	19	8	7		
Mean (SD)	8.77 (18.732)	0.00 (22.222)	4.17 (11.785)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 33.3	0.0, 0.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Appetite Loss

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	23	23	13	12		
Mean (SD)	10.14 (25.495)	2.90 (30.005)	17.95 (35.001)	5.56 (19.245)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 33.3		
Week 24						
n	20	20	13	12		
Mean (SD)	18.33 (31.484)	11.67 (34.666)	15.38 (22.008)	2.78 (9.623)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 66.7	0.0, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Appetite Loss

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	12	12	30	30
Mean (SD)	9.26 (15.363)	1.85 (17.977)	22.22 (32.824)	8.33 (35.176)	14.44 (24.264)	4.44 (25.869)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0
Week 56						
n	12	12	11	10	23	22
Mean (SD)	8.33 (15.076)	5.56 (19.245)	6.06 (13.484)	-10.00 (22.498)	7.25 (14.058)	-1.52 (21.767)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Appetite Loss

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	8	7	16	15
Mean (SD)	0.00 (0.000)	0.00 (0.000)	20.83 (24.801)	4.76 (29.991)	10.42 (20.069)	2.22 (19.787)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-33.3, 66.7
Week 88						
n	6	6	5	5	11	11
Mean (SD)	5.56 (13.608)	5.56 (13.608)	13.33 (18.257)	-6.67 (36.515)	9.09 (15.570)	0.00 (25.820)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Appetite Loss

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	2	2	3	3
Mean (SD)	0.00 (NA)	0.00 (NA)	66.67 (0.000)	16.67 (23.570)	44.44 (38.490)	11.11 (19.245)
Median	0.00	0.00	66.67	16.67	66.67	0.00
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	0.0, 33.3	0.0, 66.7	0.0, 33.3

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Constipation

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	25		12		37	
Mean (SD)	12.00 (21.257)		13.89 (17.164)		12.61 (19.800)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 33.3		0.0, 66.7	
Week 2						
n	24	24	13	12		
Mean (SD)	9.72 (23.008)	-2.78 (19.453)	15.38 (29.235)	0.00 (28.427)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Constipation

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	25	25	13	12		
Mean (SD)	5.33 (15.753)	-6.67 (19.245)	20.51 (28.991)	8.33 (25.126)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		
Week 8						
n	19	19	8	7		
Mean (SD)	3.51 (10.510)	-3.51 (15.294)	16.67 (25.198)	9.52 (16.265)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	0.0, 33.3		

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Constipation

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	23	23	13	12		
Mean (SD)	5.80 (21.678)	-7.25 (19.991)	17.95 (32.247)	5.56 (23.925)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		
Week 24						
n	20	20	13	12		
Mean (SD)	3.33 (14.907)	-8.33 (18.337)	15.38 (22.008)	2.78 (17.164)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 66.7	-33.3, 33.3		

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Modified Randomized Set

Constipation

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	12	12	30	30
Mean (SD)	5.56 (17.150)	-11.11 (22.866)	16.67 (22.473)	2.78 (17.164)	10.00 (19.865)	-5.56 (21.591)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-66.7, 33.3
Week 56						
n	12	12	11	10	23	22
Mean (SD)	11.11 (21.711)	-8.33 (25.126)	18.18 (22.918)	0.00 (15.713)	14.49 (22.079)	-4.55 (21.320)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-66.7, 33.3

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Constipation

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	8	7	16	15
Mean (SD)	12.50 (17.252)	-8.33 (23.570)	16.67 (17.817)	4.76 (23.002)	14.58 (17.078)	-2.22 (23.458)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3
Week 88						
n	6	6	5	5	11	11
Mean (SD)	11.11 (17.213)	-11.11 (17.213)	40.00 (54.772)	26.67 (54.772)	24.24 (39.696)	6.06 (41.682)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Constipation

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	2	2	3	3
Mean (SD)	33.33 (NA)	-33.33 (NA)	33.33 (47.140)	0.00 (47.140)	33.33 (33.333)	-11.11 (38.490)
Median	33.33	-33.33	33.33	0.00	33.33	-33.33
Min, Max	33.3, 33.3	-33.3, -33.3	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Diarrhea

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	25		12		37	
Mean (SD)	2.67 (9.230)		19.44 (26.432)		8.11 (18.267)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 33.3		0.0, 66.7		0.0, 66.7	
Week 2						
n	24	24	13	12		
Mean (SD)	5.56 (21.234)	2.78 (21.795)	2.56 (9.245)	-16.67 (22.473)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 33.3	-66.7, 0.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Diarrhea

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	25	25	13	12		
Mean (SD)	5.33 (15.753)	2.67 (19.052)	2.56 (9.245)	-16.67 (22.473)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 33.3	-66.7, 0.0		
Week 8						
n	19	19	8	7		
Mean (SD)	7.02 (13.962)	5.26 (12.488)	12.50 (24.801)	-14.29 (17.817)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 66.7	-33.3, 0.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Diarrhea

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	23	23	13	12		
Mean (SD)	4.35 (11.478)	1.45 (12.220)	5.13 (18.490)	-13.89 (36.121)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-66.7, 66.7		
Week 24						
n	20	20	13	12		
Mean (SD)	1.67 (7.454)	1.67 (7.454)	7.69 (14.618)	-11.11 (21.711)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	-66.7, 0.0		

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Modified Randomized Set

Diarrhea

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	12	12	30	30
Mean (SD)	3.70 (10.779)	0.00 (16.169)	5.56 (12.975)	-13.89 (26.432)	4.44 (11.525)	-5.56 (21.591)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3
Week 56						
n	12	12	11	10	23	22
Mean (SD)	2.78 (9.623)	2.78 (9.623)	9.09 (15.570)	-16.67 (28.328)	5.80 (12.918)	-6.06 (22.150)
Median	0.00	0.00	0.00	-16.67	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Diarrhea

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	8	7	16	15
Mean (SD)	0.00 (0.000)	-4.17 (11.785)	20.83 (24.801)	-4.76 (23.002)	10.42 (20.069)	-4.44 (17.213)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3
Week 88						
n	6	6	5	5	11	11
Mean (SD)	5.56 (13.608)	5.56 (13.608)	6.67 (14.907)	-13.33 (38.006)	6.06 (13.484)	-3.03 (27.707)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Diarrhea

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	2	2	3	3
Mean (SD)	0.00 (NA)	0.00 (NA)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Financial Difficulties

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	25		12		37	
Mean (SD)	12.00 (25.240)		27.78 (39.781)		17.12 (31.053)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	25	25	13	12		
Mean (SD)	12.00 (30.246)	0.00 (13.608)	28.21 (42.701)	2.78 (36.121)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 100.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
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Modified Randomized Set

Financial Difficulties

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	25	25	13	12		
Mean (SD)	14.67 (28.996)	2.67 (21.344)	25.64 (43.363)	0.00 (37.605)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 100.0		
Week 8						
n	19	19	8	7		
Mean (SD)	3.51 (10.510)	-3.51 (10.510)	4.17 (11.785)	-4.76 (12.599)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 0.0		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties. Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem. Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan. D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point. Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Financial Difficulties

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	23	23	13	12		
Mean (SD)	13.04 (27.959)	1.45 (6.950)	28.21 (42.701)	2.78 (36.121)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	0.0, 33.3	0.0, 100.0	-66.7, 100.0		
Week 24						
n	20	20	13	12		
Mean (SD)	15.00 (33.289)	5.00 (24.839)	25.64 (43.363)	0.00 (37.605)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-66.7, 100.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Financial Difficulties

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	12	12	30	30
Mean (SD)	11.11 (22.866)	-1.85 (21.305)	22.22 (38.490)	-5.56 (44.571)	15.56 (29.985)	-3.33 (31.984)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-100.0, 100.0	0.0, 100.0	-100.0, 100.0
Week 56						
n	12	12	11	10	23	22
Mean (SD)	8.33 (28.868)	-5.56 (19.245)	30.30 (37.873)	0.00 (47.140)	18.84 (34.562)	-3.03 (33.976)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 0.0	0.0, 100.0	-66.7, 100.0	0.0, 100.0	-66.7, 100.0

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Financial Difficulties

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	8	7	16	15
Mean (SD)	12.50 (17.252)	0.00 (17.817)	12.50 (24.801)	-14.29 (26.227)	12.50 (20.638)	-6.67 (22.537)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-66.7, 0.0	0.0, 66.7	-66.7, 33.3
Week 88						
n	6	6	5	5	11	11
Mean (SD)	0.00 (0.000)	-11.11 (27.217)	6.67 (14.907)	-26.67 (36.515)	3.03 (10.050)	-18.18 (31.140)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Financial Difficulties

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	2	2	3	3
Mean (SD)	0.00 (NA)	0.00 (NA)	16.67 (23.570)	-66.67 (0.000)	11.11 (19.245)	-44.44 (38.490)
Median	0.00	0.00	16.67	-66.67	0.00	-66.67
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 33.3	-66.7, -66.7	0.0, 33.3	-66.7, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Fatigue

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	42.71 (29.344)		49.07 (30.136)		44.44 (29.348)	
Median	33.33		50.00		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	31	31	12	11		
Mean (SD)	30.82 (21.220)	-12.54 (20.636)	40.74 (22.390)	-7.07 (25.470)		
Median	33.33	-11.11	33.33	0.00		
Min, Max	0.0, 77.8	-66.7, 22.2	11.1, 100.0	-44.4, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Fatigue

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	32	32	12	12		
Mean (SD)	27.08 (20.534)	-15.63 (23.249)	46.30 (23.130)	-2.78 (24.675)		
Median	27.78	-11.11	33.33	5.56		
Min, Max	0.0, 77.8	-66.7, 22.2	11.1, 88.9	-66.7, 22.2		
Week 8						
n	26	26	11	11		
Mean (SD)	31.62 (20.534)	-11.97 (23.502)	32.32 (17.536)	-15.15 (24.980)		
Median	33.33	0.00	33.33	-11.11		
Min, Max	0.0, 77.8	-66.7, 22.2	0.0, 55.6	-77.8, 11.1		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Fatigue

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	29	29	12	12		
Mean (SD)	25.67 (21.640)	-15.71 (23.291)	36.11 (15.076)	-12.96 (26.308)		
Median	22.22	-11.11	33.33	-11.11		
Min, Max	0.0, 66.7	-66.7, 22.2	11.1, 66.7	-77.8, 22.2		
Week 24						
n	29	29	11	11		
Mean (SD)	21.84 (16.925)	-19.54 (26.168)	33.33 (25.337)	-11.11 (19.876)		
Median	22.22	-11.11	33.33	0.00		
Min, Max	0.0, 66.7	-77.8, 33.3	0.0, 88.9	-44.4, 22.2		

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Modified Randomized Set

Fatigue

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	28	28	9	9	37	37
Mean (SD)	20.63 (15.920)	-18.25 (24.607)	41.98 (34.146)	0.00 (20.031)	25.83 (23.138)	-13.81 (24.624)
Median	22.22	-11.11	33.33	0.00	22.22	-11.11
Min, Max	0.0, 55.6	-77.8, 11.1	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-77.8, 33.3
Week 56						
n	22	22	6	6	28	28
Mean (SD)	28.28 (19.007)	-10.10 (26.541)	33.33 (15.713)	-7.41 (21.849)	29.37 (18.198)	-9.52 (25.249)
Median	33.33	0.00	33.33	-16.67	33.33	-5.56
Min, Max	0.0, 66.7	-66.7, 33.3	11.1, 55.6	-22.2, 33.3	0.0, 66.7	-66.7, 33.3

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Modified Randomized Set

Fatigue

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	4	4	17	17
Mean (SD)	23.08 (18.404)	-22.22 (24.845)	44.44 (15.713)	-11.11 (15.713)	28.10 (19.688)	-19.61 (23.084)
Median	22.22	-11.11	38.89	-5.56	33.33	-11.11
Min, Max	0.0, 66.7	-66.7, 22.2	33.3, 66.7	-33.3, 0.0	0.0, 66.7	-66.7, 22.2
Week 88						
n	8	8	3	3	11	11
Mean (SD)	22.22 (22.222)	-16.67 (23.002)	29.63 (27.962)	-25.93 (12.830)	24.24 (22.672)	-19.19 (20.543)
Median	16.67	-11.11	33.33	-33.33	22.22	-22.22
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 55.6	-33.3, -11.1	0.0, 66.7	-66.7, 0.0

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Fatigue

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	0	0	3	3
Mean (SD)	7.41 (12.830)	-14.81 (16.973)			7.41 (12.830)	-14.81 (16.973)
Median	0.00	-11.11			0.00	-11.11
Min, Max	0.0, 22.2	-33.3, 0.0			0.0, 22.2	-33.3, 0.0

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Nausea and Vomiting

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	4.17 (13.387)		8.33 (19.462)		5.30 (15.153)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	31	31	12	11		
Mean (SD)	2.15 (5.680)	-2.15 (11.974)	8.33 (16.667)	-3.03 (10.050)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 16.7	-50.0, 16.7	0.0, 50.0	-16.7, 16.7		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Nausea and Vomiting

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	32	32	12	12		
Mean (SD)	2.08 (5.600)	-2.08 (11.785)	4.17 (10.360)	-4.17 (20.257)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 16.7	-50.0, 16.7	0.0, 33.3	-66.7, 16.7		
Week 8						
n	26	26	11	11		
Mean (SD)	7.69 (18.397)	2.56 (20.919)	6.06 (15.407)	-3.03 (23.355)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 83.3	-33.3, 83.3	0.0, 50.0	-66.7, 33.3		

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Modified Randomized Set

Nausea and Vomiting

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	29	29	12	12		
Mean (SD)	3.45 (9.322)	-0.57 (12.185)	1.39 (4.811)	-6.94 (19.408)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-50.0, 33.3	0.0, 16.7	-66.7, 0.0		
Week 24						
n	29	29	11	11		
Mean (SD)	1.72 (5.166)	-2.30 (10.657)	1.52 (5.025)	-1.52 (8.989)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 16.7	-50.0, 16.7	0.0, 16.7	-16.7, 16.7		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Nausea and Vomiting

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	28	28	9	9	37	37
Mean (SD)	2.98 (6.500)	0.60 (5.522)	1.85 (5.556)	0.00 (8.333)	2.70 (6.228)	0.45 (6.194)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	-16.7, 16.7	0.0, 16.7	-16.7, 16.7	0.0, 16.7	-16.7, 16.7
Week 56						
n	22	22	6	6	28	28
Mean (SD)	0.76 (3.553)	-2.27 (9.337)	2.78 (6.804)	0.00 (0.000)	1.19 (4.371)	-1.79 (8.289)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	-33.3, 16.7	0.0, 16.7	0.0, 0.0	0.0, 16.7	-33.3, 16.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Nausea and Vomiting

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	4	4	17	17
Mean (SD)	1.28 (4.623)	-1.28 (8.226)	4.17 (8.333)	0.00 (13.608)	1.96 (5.535)	-0.98 (9.262)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	-16.7, 16.7	0.0, 16.7	-16.7, 16.7	0.0, 16.7	-16.7, 16.7
Week 88						
n	8	8	3	3	11	11
Mean (SD)	2.08 (5.893)	0.00 (0.000)	11.11 (9.623)	5.56 (9.623)	4.55 (7.785)	1.52 (5.025)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 16.7	0.0, 0.0	0.0, 16.7	0.0, 16.7	0.0, 16.7	0.0, 16.7

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Nausea and Vomiting

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	0	0	3	3
Mean (SD)	0.00 (0.000)	0.00 (0.000)			0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Pain

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	14.06 (22.840)		19.44 (22.285)		15.53 (22.561)	
Median	0.00		16.67		0.00	
Min, Max	0.0, 83.3		0.0, 66.7		0.0, 83.3	
Week 2						
n	31	31	12	11		
Mean (SD)	11.29 (18.453)	-3.23 (17.437)	23.61 (20.669)	0.00 (14.907)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-33.3, 16.7		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Pain

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	32	32	12	12		
Mean (SD)	10.94 (16.181)	-3.13 (19.601)	15.28 (21.856)	-4.17 (14.434)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 50.0	-66.7, 50.0	0.0, 66.7	-33.3, 16.7		
Week 8						
n	26	26	11	11		
Mean (SD)	18.59 (29.184)	6.41 (33.690)	12.12 (15.076)	-9.09 (20.226)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 33.3	-33.3, 16.7		

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Pain

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	29	29	12	12		
Mean (SD)	9.77 (19.168)	-3.45 (24.144)	19.44 (17.164)	0.00 (20.101)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 66.7	-83.3, 33.3	0.0, 50.0	-33.3, 33.3		
Week 24						
n	29	29	11	11		
Mean (SD)	9.20 (17.586)	-4.02 (23.844)	16.67 (25.820)	-1.52 (17.408)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-83.3, 33.3	0.0, 83.3	-33.3, 16.7		

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Pain

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	28	28	9	9	37	37
Mean (SD)	10.12 (17.766)	-4.17 (22.962)	20.37 (33.101)	9.26 (38.289)	12.61 (22.363)	-0.90 (27.483)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-83.3, 33.3	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-83.3, 100.0
Week 56						
n	22	22	6	6	28	28
Mean (SD)	14.39 (21.390)	-3.03 (29.830)	25.00 (29.345)	13.89 (37.143)	16.67 (23.130)	0.60 (31.584)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 66.7	-83.3, 66.7	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-83.3, 66.7

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Pain

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	4	4	16	16
Mean (SD)	8.33 (11.237)	-9.72 (22.983)	8.33 (16.667)	0.00 (27.217)	8.33 (12.172)	-7.29 (23.546)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-50.0, 16.7	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-50.0, 33.3
Week 88						
n	8	8	3	3	11	11
Mean (SD)	8.33 (12.599)	-10.42 (25.099)	16.67 (28.868)	5.56 (41.944)	10.61 (17.115)	-6.06 (29.129)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 16.7	0.0, 50.0	-33.3, 50.0	0.0, 50.0	-66.7, 50.0

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Pain

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	0	0	3	3
Mean (SD)	0.00 (0.000)	0.00 (0.000)			0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Dyspnoea

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	37.50 (27.760)		33.33 (34.816)		36.36 (29.481)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	31	31	12	11		
Mean (SD)	23.66 (26.096)	-15.05 (24.097)	33.33 (31.782)	0.00 (25.820)		
Median	33.33	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Dyspnoea

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	32	32	12	12		
Mean (SD)	15.63 (23.924)	-21.88 (26.246)	36.11 (30.011)	2.78 (33.207)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 66.7		
Week 8						
n	26	26	11	11		
Mean (SD)	17.95 (23.534)	-20.51 (26.795)	21.21 (22.473)	-15.15 (34.524)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 66.7	-100.0, 33.3		

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Dyspnoea

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	29	29	12	12		
Mean (SD)	14.94 (21.056)	-21.84 (22.318)	19.44 (22.285)	-13.89 (43.712)		
Median	0.00	-33.33	16.67	0.00		
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 66.7	-100.0, 66.7		
Week 24						
n	29	29	11	11		
Mean (SD)	14.94 (21.056)	-21.84 (25.628)	12.12 (16.817)	-15.15 (31.140)		
Median	0.00	-33.33	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 33.3	-66.7, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Dyspnoea

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	28	28	9	9	37	37
Mean (SD)	11.90 (20.716)	-22.62 (20.394)	11.11 (16.667)	-18.52 (24.216)	11.71 (19.589)	-21.62 (21.106)
Median	0.00	-33.33	0.00	0.00	0.00	-33.33
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 66.7	-66.7, 0.0
Week 56						
n	22	22	6	6	28	28
Mean (SD)	16.67 (22.420)	-16.67 (22.420)	11.11 (17.213)	-5.56 (25.092)	15.48 (21.242)	-14.29 (23.002)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-66.7, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Dyspnoea

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	4	4	17	17
Mean (SD)	7.69 (14.618)	-33.33 (19.245)	0.00 (0.000)	-25.00 (31.914)	5.88 (13.098)	-31.37 (21.955)
Median	0.00	-33.33	0.00	-16.67	0.00	-33.33
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 0.0	-66.7, 0.0	0.0, 33.3	-66.7, 0.0
Week 88						
n	8	8	3	3	11	11
Mean (SD)	12.50 (24.801)	-25.00 (23.570)	0.00 (0.000)	-22.22 (38.490)	9.09 (21.556)	-24.24 (26.208)
Median	0.00	-33.33	0.00	0.00	0.00	-33.33
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 0.0	-66.7, 0.0	0.0, 66.7	-66.7, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Dyspnoea

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	0	0	3	3
Mean (SD)	0.00 (0.000)	-11.11 (19.245)			0.00 (0.000)	-11.11 (19.245)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0			0.0, 0.0	-33.3, 0.0

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Insomnia

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	25.00 (29.329)		25.00 (25.126)		25.00 (27.958)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	31	31	12	11		
Mean (SD)	21.51 (29.248)	-3.23 (17.961)	25.00 (28.868)	0.00 (25.820)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 33.3		

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Insomnia

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	32	32	12	12		
Mean (SD)	16.67 (29.329)	-8.33 (22.401)	16.67 (30.151)	-8.33 (25.126)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 33.3		
Week 8						
n	26	26	11	11		
Mean (SD)	10.26 (15.689)	-15.38 (23.534)	24.24 (30.151)	0.00 (25.820)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 100.0	-33.3, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Insomnia

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	29	29	12	12		
Mean (SD)	14.94 (24.537)	-10.34 (22.009)	25.00 (20.719)	0.00 (24.618)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	-33.3, 33.3		
Week 24						
n	29	29	11	11		
Mean (SD)	14.94 (24.537)	-10.34 (29.685)	18.18 (22.918)	-6.06 (25.025)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-100.0, 66.7	0.0, 66.7	-66.7, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Insomnia

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	28	28	9	9	37	37
Mean (SD)	15.48 (24.816)	-8.33 (23.351)	29.63 (35.136)	3.70 (35.136)	18.92 (27.823)	-5.41 (26.659)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7
Week 56						
n	22	22	6	6	28	28
Mean (SD)	21.21 (26.318)	0.00 (23.002)	44.44 (34.427)	11.11 (27.217)	26.19 (29.197)	2.38 (23.880)
Median	16.67	0.00	33.33	16.67	33.33	0.00
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 66.7

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Insomnia

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	4	4	17	17
Mean (SD)	12.82 (21.681)	-10.26 (21.014)	50.00 (43.033)	0.00 (47.140)	21.57 (31.049)	-7.84 (27.712)
Median	0.00	0.00	50.00	16.67	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 33.3
Week 88						
n	8	8	3	3	11	11
Mean (SD)	12.50 (24.801)	-4.17 (11.785)	44.44 (50.918)	-11.11 (50.918)	21.21 (34.230)	-6.06 (25.025)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Insomnia

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	0	0	3	3
Mean (SD)	11.11 (19.245)	0.00 (0.000)			11.11 (19.245)	0.00 (0.000)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 33.3	0.0, 0.0			0.0, 33.3	0.0, 0.0

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Appetite Loss

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	10.42 (17.835)		22.22 (32.824)		13.64 (23.092)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 100.0		0.0, 100.0	
Week 2						
n	31	31	12	11		
Mean (SD)	8.60 (17.144)	-2.15 (17.074)	22.22 (21.711)	-3.03 (17.979)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3		

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Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	32	32	12	12		
Mean (SD)	7.29 (16.361)	-3.13 (15.518)	16.67 (26.591)	-5.56 (23.925)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-66.7, 33.3		
Week 8						
n	26	26	11	11		
Mean (SD)	16.67 (19.437)	3.85 (19.612)	18.18 (22.918)	-6.06 (35.957)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-100.0, 33.3		

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Appetite Loss

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	29	29	12	12		
Mean (SD)	4.60 (11.698)	-4.60 (19.363)	16.67 (22.473)	-5.56 (27.828)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-66.7, 33.3		
Week 24						
n	29	29	11	11		
Mean (SD)	5.75 (12.814)	-3.45 (22.440)	18.18 (27.340)	3.03 (17.979)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-33.3, 33.3		

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Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	28	28	9	9	37	37
Mean (SD)	4.76 (11.878)	-2.38 (15.525)	14.81 (17.568)	3.70 (11.111)	7.21 (13.911)	-0.90 (14.670)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3	-33.3, 33.3
Week 56						
n	22	22	6	6	28	28
Mean (SD)	7.58 (14.298)	0.00 (20.574)	22.22 (40.369)	11.11 (27.217)	10.71 (22.321)	2.38 (22.090)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	0.0, 66.7	0.0, 100.0	-33.3, 66.7

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Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	4	4	17	17
Mean (SD)	5.13 (12.518)	-7.69 (14.618)	16.67 (19.245)	0.00 (0.000)	7.84 (14.575)	-5.88 (13.098)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	0.0, 0.0	0.0, 33.3	-33.3, 0.0
Week 88						
n	8	8	3	3	11	11
Mean (SD)	8.33 (15.430)	-4.17 (11.785)	22.22 (19.245)	11.11 (19.245)	12.12 (16.817)	0.00 (14.907)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	0.0, 33.3	0.0, 33.3	-33.3, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Appetite Loss

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	0	0	3	3
Mean (SD)	0.00 (0.000)	0.00 (0.000)			0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Constipation

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	14.58 (25.312)		8.33 (20.719)		12.88 (24.076)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	31	31	12	11		
Mean (SD)	9.68 (23.084)	-5.38 (19.430)	19.44 (33.207)	12.12 (22.473)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	0.0, 66.7		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Constipation

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	32	32	12	12		
Mean (SD)	13.54 (26.585)	-1.04 (23.163)	16.67 (30.151)	8.33 (25.126)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-33.3, 66.7		
Week 8						
n	26	26	11	11		
Mean (SD)	7.69 (17.147)	-8.97 (24.143)	15.15 (34.524)	6.06 (13.484)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	0.0, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Constipation

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	29	29	12	12		
Mean (SD)	12.64 (25.841)	-1.15 (20.860)	16.67 (33.333)	8.33 (20.719)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 100.0	0.0, 66.7		
Week 24						
n	29	29	11	11		
Mean (SD)	9.20 (21.633)	-4.60 (21.314)	18.18 (31.140)	9.09 (26.208)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 66.7		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Constipation

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	28	28	9	9	37	37
Mean (SD)	7.14 (13.929)	-7.14 (20.998)	14.81 (24.216)	7.41 (27.778)	9.01 (16.940)	-3.60 (23.285)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-66.7, 66.7
Week 56						
n	22	22	6	6	28	28
Mean (SD)	9.09 (23.417)	-7.58 (17.614)	0.00 (0.000)	0.00 (0.000)	7.14 (20.998)	-5.95 (15.853)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 0.0	0.0, 0.0	0.0, 0.0	0.0, 100.0	-66.7, 0.0

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Constipation

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	4	4	17	17
Mean (SD)	12.82 (28.991)	-7.69 (19.971)	0.00 (0.000)	0.00 (0.000)	9.80 (25.725)	-5.88 (17.620)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 0.0	0.0, 0.0	0.0, 100.0	-33.3, 33.3
Week 88						
n	8	8	3	3	11	11
Mean (SD)	16.67 (17.817)	0.00 (25.198)	0.00 (0.000)	0.00 (0.000)	12.12 (16.817)	0.00 (21.082)
Median	16.67	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 0.0	0.0, 0.0	0.0, 33.3	-33.3, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Constipation

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	0	0	3	3
Mean (SD)	11.11 (19.245)	0.00 (0.000)			11.11 (19.245)	0.00 (0.000)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 33.3	0.0, 0.0			0.0, 33.3	0.0, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Diarrhea

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	7.29 (20.275)		5.56 (12.975)		6.82 (18.440)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 33.3		0.0, 100.0	
Week 2						
n	32	32	12	11		
Mean (SD)	6.25 (15.698)	-1.04 (13.345)	8.33 (20.719)	3.03 (17.979)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-33.3, 33.3		

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Diarrhea

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	32	32	12	12		
Mean (SD)	5.21 (12.297)	-2.08 (20.631)	5.56 (12.975)	0.00 (14.213)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	-33.3, 33.3		
Week 8						
n	26	26	11	11		
Mean (SD)	7.69 (17.147)	0.00 (23.094)	3.03 (10.050)	-3.03 (17.979)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 66.7	0.0, 33.3	-33.3, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Diarrhea

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	29	29	12	12		
Mean (SD)	6.90 (13.742)	-1.15 (18.862)	2.78 (9.623)	-2.78 (17.164)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-33.3, 33.3		
Week 24						
n	29	29	11	11		
Mean (SD)	8.05 (14.516)	0.00 (19.920)	0.00 (0.000)	-3.03 (10.050)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 0.0	-33.3, 0.0		

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Table GERMANY.2.5.4.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Diarrhea

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	28	28	9	9	37	37
Mean (SD)	5.95 (13.001)	-2.38 (23.880)	11.11 (16.667)	7.41 (14.699)	7.21 (13.911)	0.00 (22.222)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3	-100.0, 33.3
Week 56						
n	22	22	6	6	28	28
Mean (SD)	9.09 (15.195)	1.52 (26.181)	5.56 (13.608)	0.00 (0.000)	8.33 (14.699)	1.19 (23.098)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	0.0, 0.0	0.0, 33.3	-100.0, 33.3

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Table GERMANY.2.5.4.4
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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Diarrhea

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	4	4	16	16
Mean (SD)	0.00 (0.000)	0.00 (0.000)	33.33 (47.140)	25.00 (50.000)	8.33 (25.820)	6.25 (25.000)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 100.0	0.0, 100.0	0.0, 100.0	0.0, 100.0
Week 88						
n	8	8	3	3	11	11
Mean (SD)	0.00 (0.000)	0.00 (0.000)	11.11 (19.245)	0.00 (0.000)	3.03 (10.050)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 33.3	0.0, 0.0	0.0, 33.3	0.0, 0.0

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Table GERMANY.2.5.4.4
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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Diarrhea

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	0	0	3	3
Mean (SD)	0.00 (0.000)	0.00 (0.000)			0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.4
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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Financial Difficulties

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	27.08 (33.266)		19.44 (22.285)		25.00 (30.605)	
Median	16.67		16.67		16.67	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	32	32	12	11		
Mean (SD)	22.92 (28.634)	-4.17 (16.396)	19.44 (22.285)	3.03 (17.979)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 66.7	-33.3, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Financial Difficulties

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	32	32	12	12		
Mean (SD)	19.79 (25.201)	-7.29 (18.422)	11.11 (16.412)	-8.33 (20.719)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-33.3, 33.3		
Week 8						
n	26	26	11	11		
Mean (SD)	19.23 (25.251)	-8.97 (27.580)	21.21 (26.968)	3.03 (23.355)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 66.7	-33.3, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Financial Difficulties

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	29	29	12	12		
Mean (SD)	21.84 (25.628)	-5.75 (21.947)	16.67 (26.591)	-2.78 (30.011)		
Median	33.33	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	-33.3, 66.7		
Week 24						
n	29	29	11	11		
Mean (SD)	19.54 (25.999)	-8.05 (24.649)	18.18 (22.918)	-3.03 (17.979)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 66.7	-33.3, 33.3		

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Financial Difficulties

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	28	28	9	9	37	37
Mean (SD)	21.43 (28.995)	-7.14 (24.607)	18.52 (24.216)	-7.41 (22.222)	20.72 (27.612)	-7.21 (23.747)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-66.7, 33.3
Week 56						
n	22	22	6	6	28	28
Mean (SD)	15.15 (19.860)	-10.61 (18.930)	16.67 (27.889)	-5.56 (25.092)	15.48 (21.242)	-9.52 (19.994)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Financial Difficulties

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	4	4	16	16
Mean (SD)	5.56 (12.975)	-16.67 (26.591)	8.33 (16.667)	-16.67 (19.245)	6.25 (13.437)	-16.67 (24.343)
Median	0.00	0.00	0.00	-16.67	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-66.7, 0.0
Week 88						
n	8	8	3	3	11	11
Mean (SD)	16.67 (17.817)	-8.33 (15.430)	11.11 (19.245)	-22.22 (19.245)	15.15 (17.408)	-12.12 (16.817)
Median	16.67	0.00	0.00	-33.33	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 0.0

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
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Financial Difficulties

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	0	0	3	3
Mean (SD)	0.00 (0.000)	-22.22 (38.490)			0.00 (0.000)	-22.22 (38.490)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	-66.7, 0.0			0.0, 0.0	-66.7, 0.0

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Fatigue

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		9		33	
Mean (SD)	44.91 (25.690)		54.32 (17.954)		47.47 (23.939)	
Median	44.44		55.56		44.44	
Min, Max	0.0, 88.9		33.3, 88.9		0.0, 88.9	
Week 2						
n	24	24	9	8		
Mean (SD)	33.80 (26.513)	-11.11 (20.198)	39.51 (19.334)	-9.72 (16.197)		
Median	33.33	-5.56	33.33	-5.56		
Min, Max	0.0, 100.0	-55.6, 22.2	11.1, 66.7	-33.3, 11.1		

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Modified Randomized Set

Fatigue

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	24	24	10	9		
Mean (SD)	28.24 (22.217)	-16.67 (20.722)	54.44 (29.374)	4.94 (16.769)		
Median	33.33	-11.11	50.00	0.00		
Min, Max	0.0, 77.8	-55.6, 22.2	11.1, 100.0	-11.1, 44.4		
Week 8						
n	19	19	7	6		
Mean (SD)	31.58 (28.027)	-13.45 (26.079)	36.51 (18.937)	-18.52 (15.181)		
Median	22.22	-11.11	33.33	-22.22		
Min, Max	0.0, 100.0	-66.7, 44.4	11.1, 66.7	-33.3, 0.0		

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Modified Randomized Set

Fatigue

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	10	9		
Mean (SD)	27.78 (21.068)	-15.66 (20.187)	43.33 (24.259)	-14.81 (15.713)		
Median	33.33	-11.11	50.00	-11.11		
Min, Max	0.0, 66.7	-55.6, 22.2	0.0, 77.8	-44.4, 11.1		
Week 24						
n	21	21	10	9		
Mean (SD)	25.40 (26.561)	-17.99 (24.464)	45.56 (29.837)	-7.41 (24.216)		
Median	22.22	-22.22	38.89	-11.11		
Min, Max	0.0, 88.9	-66.7, 33.3	0.0, 100.0	-44.4, 44.4		

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Modified Randomized Set

Fatigue

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	8	8	27	27
Mean (SD)	26.32 (26.244)	-14.04 (20.233)	56.94 (31.110)	6.94 (21.362)	35.39 (30.668)	-7.82 (22.396)
Median	22.22	-11.11	50.00	0.00	33.33	0.00
Min, Max	0.0, 77.8	-55.6, 22.2	22.2, 100.0	-11.1, 44.4	0.0, 100.0	-55.6, 44.4
Week 56						
n	13	13	7	6	20	19
Mean (SD)	29.91 (27.735)	-8.55 (27.275)	38.10 (14.138)	-12.96 (10.924)	32.78 (23.769)	-9.94 (23.098)
Median	33.33	-22.22	33.33	-16.67	33.33	-22.22
Min, Max	0.0, 66.7	-44.4, 44.4	22.2, 66.7	-22.2, 0.0	0.0, 66.7	-44.4, 44.4

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Fatigue

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	6	5	15	14
Mean (SD)	32.10 (21.114)	-13.58 (17.372)	40.74 (13.456)	-8.89 (14.487)	35.56 (18.402)	-11.90 (15.992)
Median	33.33	-11.11	33.33	0.00	33.33	-11.11
Min, Max	0.0, 66.7	-33.3, 22.2	33.3, 66.7	-33.3, 0.0	0.0, 66.7	-33.3, 22.2
Week 88						
n	7	7	3	3	10	10
Mean (SD)	14.29 (21.956)	-25.40 (18.937)	37.04 (6.415)	-18.52 (16.973)	21.11 (21.244)	-23.33 (17.724)
Median	0.00	-33.33	33.33	-22.22	22.22	-27.78
Min, Max	0.0, 55.6	-44.4, 0.0	33.3, 44.4	-33.3, 0.0	0.0, 55.6	-44.4, 0.0

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Modified Randomized Set

Fatigue

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	18.52 (16.973)	-22.22 (29.397)	33.33 (NA)	0.00 (NA)	22.22 (15.713)	-16.67 (26.450)
Median	22.22	-11.11	33.33	0.00	27.78	-5.56
Min, Max	0.0, 33.3	-55.6, 0.0	33.3, 33.3	0.0, 0.0	0.0, 33.3	-55.6, 0.0

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Modified Randomized Set

Nausea and Vomiting

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		9		33	
Mean (SD)	0.00 (0.000)		3.70 (7.349)		1.01 (4.038)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 0.0		0.0, 16.7		0.0, 16.7	
Week 2						
n	24	24	9	8		
Mean (SD)	4.17 (11.261)	4.17 (11.261)	5.56 (11.785)	2.08 (10.681)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 50.0	0.0, 50.0	0.0, 33.3	-16.7, 16.7		

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Modified Randomized Set

Nausea and Vomiting

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	24	24	10	9		
Mean (SD)	3.47 (8.483)	3.47 (8.483)	5.00 (8.051)	1.85 (5.556)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 16.7	0.0, 16.7		
Week 8						
n	19	19	7	6		
Mean (SD)	6.14 (19.413)	6.14 (19.413)	4.76 (8.133)	2.78 (6.804)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 83.3	0.0, 83.3	0.0, 16.7	0.0, 16.7		

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Nausea and Vomiting

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	10	9		
Mean (SD)	2.27 (7.793)	2.27 (7.793)	6.67 (16.102)	-1.85 (10.015)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 50.0	-16.7, 16.7		
Week 24						
n	21	21	10	9		
Mean (SD)	1.59 (7.274)	1.59 (7.274)	3.33 (7.027)	0.00 (11.785)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 16.7	-16.7, 16.7		

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> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	8	8	27	27
Mean (SD)	4.39 (10.890)	4.39 (10.890)	2.08 (5.893)	0.00 (8.909)	3.70 (9.623)	3.09 (10.374)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 16.7	-16.7, 16.7	0.0, 33.3	-16.7, 33.3
Week 56						
n	13	13	7	6	20	19
Mean (SD)	0.00 (0.000)	0.00 (0.000)	19.05 (36.551)	19.44 (32.347)	6.67 (22.556)	6.14 (19.413)
Median	0.00	0.00	0.00	8.33	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 100.0	0.0, 83.3	0.0, 100.0	0.0, 83.3

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Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Nausea and Vomiting

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	6	5	15	14
Mean (SD)	1.85 (5.556)	1.85 (5.556)	2.78 (6.804)	3.33 (7.454)	2.22 (5.864)	2.38 (6.052)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	0.0, 16.7	0.0, 16.7	0.0, 16.7	0.0, 16.7	0.0, 16.7
Week 88						
n	7	7	3	3	10	10
Mean (SD)	0.00 (0.000)	0.00 (0.000)	11.11 (9.623)	11.11 (9.623)	3.33 (7.027)	3.33 (7.027)
Median	0.00	0.00	16.67	16.67	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 16.7	0.0, 16.7	0.0, 16.7	0.0, 16.7

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Nausea and Vomiting

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	0.00 (0.000)	0.00 (0.000)	33.33 (NA)	33.33 (NA)	8.33 (16.667)	8.33 (16.667)
Median	0.00	0.00	33.33	33.33	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	33.3, 33.3	33.3, 33.3	0.0, 33.3	0.0, 33.3

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Modified Randomized Set

Pain

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		9		33	
Mean (SD)	10.42 (26.381)		22.22 (37.268)		13.64 (29.596)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	24	24	9	8		
Mean (SD)	12.50 (28.763)	2.08 (16.530)	25.93 (26.498)	2.08 (18.767)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 66.7	-33.3, 33.3		

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Table GERMANY.2.5.4.5

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Modified Randomized Set

Pain

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	24	24	10	9		
Mean (SD)	11.81 (26.228)	1.39 (11.955)	16.67 (31.427)	-3.70 (13.889)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-16.7, 50.0	0.0, 83.3	-33.3, 16.7		
Week 8						
n	19	19	7	6		
Mean (SD)	12.28 (27.127)	8.77 (28.528)	9.52 (13.113)	-8.33 (13.944)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 33.3	-33.3, 0.0		

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Pain

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	10	9		
Mean (SD)	9.85 (24.484)	-1.52 (13.520)	23.33 (28.545)	0.00 (22.048)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 83.3	-33.3, 33.3		
Week 24						
n	21	21	10	9		
Mean (SD)	11.90 (25.355)	0.00 (15.811)	13.33 (26.989)	-7.41 (26.498)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-50.0, 33.3	0.0, 83.3	-66.7, 16.7		

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Modified Randomized Set

Pain

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	8	8	27	27
Mean (SD)	16.67 (27.778)	4.39 (10.890)	35.42 (42.199)	18.75 (37.201)	22.22 (33.011)	8.64 (22.347)
Median	0.00	0.00	25.00	0.00	0.00	0.00
Min, Max	0.0, 83.3	-16.7, 33.3	0.0, 100.0	-16.7, 100.0	0.0, 100.0	-16.7, 100.0
Week 56						
n	13	13	7	6	20	19
Mean (SD)	24.36 (33.065)	7.69 (23.189)	30.95 (29.547)	11.11 (32.773)	26.67 (31.251)	8.77 (25.681)
Median	0.00	0.00	16.67	8.33	16.67	0.00
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 66.7	-33.3, 66.7	0.0, 100.0	-33.3, 66.7

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Pain

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	6	5	14	13
Mean (SD)	10.42 (23.465)	-2.08 (13.909)	13.89 (16.387)	6.67 (14.907)	11.90 (20.073)	1.28 (14.372)
Median	0.00	0.00	8.33	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 16.7	0.0, 33.3	0.0, 33.3	0.0, 66.7	-33.3, 33.3
Week 88						
n	7	7	3	3	10	10
Mean (SD)	9.52 (25.198)	-4.76 (12.599)	22.22 (25.459)	22.22 (25.459)	13.33 (24.595)	3.33 (20.488)
Median	0.00	0.00	16.67	16.67	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 50.0	0.0, 50.0	0.0, 66.7	-33.3, 50.0

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Table GERMANY.2.5.4.5

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Modified Randomized Set

Pain

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	11.11 (19.245)	-22.22 (38.490)	16.67 (NA)	16.67 (NA)	12.50 (15.957)	-12.50 (36.956)
Median	0.00	0.00	16.67	16.67	8.33	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	16.7, 16.7	16.7, 16.7	0.0, 33.3	-66.7, 16.7

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Modified Randomized Set

Dyspnoea

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		9		33	
Mean (SD)	36.11 (29.352)		29.63 (26.058)		34.34 (28.241)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	24	24	9	8		
Mean (SD)	23.61 (28.622)	-12.50 (25.656)	22.22 (16.667)	-8.33 (23.570)		
Median	33.33	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-33.3, 33.3		

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Dyspnoea

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	24	24	10	9		
Mean (SD)	18.06 (25.968)	-18.06 (27.766)	23.33 (22.498)	-3.70 (30.932)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	-33.3, 66.7		
Week 8						
n	19	19	7	6		
Mean (SD)	26.32 (26.244)	-8.77 (24.450)	19.05 (17.817)	-11.11 (17.213)		
Median	33.33	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 33.3	-33.3, 0.0		

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Dyspnoea

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	10	9		
Mean (SD)	16.67 (19.920)	-19.70 (26.546)	30.00 (18.922)	0.00 (33.333)		
Median	0.00	-16.67	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-33.3, 66.7		
Week 24						
n	21	21	10	9		
Mean (SD)	17.46 (22.655)	-20.63 (24.667)	16.67 (17.568)	-11.11 (23.570)		
Median	0.00	-33.33	16.67	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 33.3	-33.3, 33.3		

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Modified Randomized Set

Dyspnoea

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	8	8	27	27
Mean (SD)	22.81 (29.508)	-10.53 (24.976)	16.67 (17.817)	-16.67 (17.817)	20.99 (26.390)	-12.35 (22.923)
Median	0.00	0.00	16.67	-16.67	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-33.3, 0.0	0.0, 100.0	-66.7, 33.3
Week 56						
n	13	13	7	6	20	19
Mean (SD)	28.21 (32.903)	-5.13 (26.688)	4.76 (12.599)	-22.22 (27.217)	20.00 (29.419)	-10.53 (27.336)
Median	33.33	0.00	0.00	-16.67	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-66.7, 0.0	0.0, 100.0	-66.7, 33.3

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Modified Randomized Set

Dyspnoea

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	6	5	15	14
Mean (SD)	18.52 (24.216)	-14.81 (17.568)	22.22 (27.217)	0.00 (33.333)	20.00 (24.560)	-9.52 (24.209)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3
Week 88						
n	7	7	3	3	10	10
Mean (SD)	0.00 (0.000)	-23.81 (25.198)	11.11 (19.245)	0.00 (33.333)	3.33 (10.541)	-16.67 (28.328)
Median	0.00	-33.33	0.00	0.00	0.00	-16.67
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-66.7, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Dyspnoea

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	0.00 (0.000)	-22.22 (38.490)	0.00 (NA)	0.00 (NA)	0.00 (0.000)	-16.67 (33.333)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	-66.7, 0.0

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Insomnia

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		9		33	
Mean (SD)	20.83 (27.474)		22.22 (16.667)		21.21 (24.746)	
Median	0.00		33.33		0.00	
Min, Max	0.0, 66.7		0.0, 33.3		0.0, 66.7	
Week 2						
n	24	24	9	8		
Mean (SD)	20.83 (33.783)	0.00 (21.979)	22.22 (33.333)	0.00 (30.861)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

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Modified Randomized Set

Insomnia

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	24	24	10	9		
Mean (SD)	20.83 (29.180)	0.00 (17.025)	26.67 (34.427)	3.70 (35.136)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 66.7		
Week 8						
n	19	19	7	6		
Mean (SD)	15.79 (20.393)	-3.51 (24.582)	4.76 (12.599)	-11.11 (17.213)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 33.3	-33.3, 0.0		

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Insomnia

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	10	9		
Mean (SD)	16.67 (24.667)	-4.55 (18.674)	23.33 (35.312)	0.00 (33.333)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-33.3, 66.7		
Week 24						
n	21	21	10	9		
Mean (SD)	14.29 (22.537)	-4.76 (26.427)	20.00 (17.213)	-3.70 (20.031)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 33.3	-33.3, 33.3		

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Modified Randomized Set

Insomnia

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	8	8	27	27
Mean (SD)	22.81 (27.336)	1.75 (20.709)	37.50 (45.207)	16.67 (35.635)	27.16 (33.381)	6.17 (26.209)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 66.7
Week 56						
n	13	13	7	6	20	19
Mean (SD)	15.38 (22.008)	2.56 (28.744)	38.10 (35.635)	16.67 (27.889)	23.33 (28.817)	7.02 (28.499)
Median	0.00	0.00	33.33	0.00	16.67	0.00
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 100.0	0.0, 66.7	0.0, 100.0	-33.3, 66.7

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Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Insomnia

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	6	5	15	14
Mean (SD)	14.81 (24.216)	-3.70 (11.111)	33.33 (29.814)	13.33 (18.257)	22.22 (27.217)	2.38 (15.821)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 66.7	0.0, 33.3	0.0, 66.7	-33.3, 33.3
Week 88						
n	7	7	3	3	10	10
Mean (SD)	4.76 (12.599)	-9.52 (16.265)	44.44 (50.918)	22.22 (38.490)	16.67 (32.394)	0.00 (27.217)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 100.0	0.0, 66.7	0.0, 100.0	-33.3, 66.7

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Insomnia

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	0.00 (0.000)	-22.22 (38.490)	100.00 (NA)	66.67 (NA)	25.00 (50.000)	0.00 (54.433)
Median	0.00	0.00	100.00	66.67	0.00	0.00
Min, Max	0.0, 0.0	-66.7, 0.0	100.0, 100.0	66.7, 66.7	0.0, 100.0	-66.7, 66.7

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Appetite Loss

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		9		33	
Mean (SD)	9.72 (18.334)		18.52 (24.216)		12.12 (20.101)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	24	24	9	8		
Mean (SD)	13.89 (23.909)	4.17 (22.656)	18.52 (17.568)	0.00 (17.817)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 33.3	-33.3, 33.3		

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Modified Randomized Set

Appetite Loss

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	24	24	10	9		
Mean (SD)	6.94 (16.966)	-2.78 (16.787)	13.33 (23.307)	-3.70 (11.111)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 0.0		
Week 8						
n	19	19	7	6		
Mean (SD)	14.04 (20.233)	1.75 (17.476)	14.29 (17.817)	-5.56 (13.608)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 33.3	-33.3, 0.0		

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Modified Randomized Set

Appetite Loss

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	10	9		
Mean (SD)	10.61 (23.874)	0.00 (29.096)	16.67 (28.328)	0.00 (23.570)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 66.7	-33.3, 33.3		
Week 24						
n	21	21	10	9		
Mean (SD)	11.11 (24.343)	1.59 (32.449)	16.67 (28.328)	0.00 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 100.0	0.0, 66.7	-33.3, 33.3		

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Modified Randomized Set

Appetite Loss

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	8	8	27	27
Mean (SD)	7.02 (13.962)	1.75 (17.476)	20.83 (24.801)	8.33 (15.430)	11.11 (18.490)	3.70 (16.879)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	0.0, 33.3	0.0, 66.7	-33.3, 33.3
Week 56						
n	13	13	7	6	20	19
Mean (SD)	7.69 (14.618)	7.69 (14.618)	9.52 (16.265)	0.00 (0.000)	8.33 (14.809)	5.26 (12.488)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 0.0	0.0, 33.3	0.0, 33.3

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Appetite Loss

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	6	5	15	14
Mean (SD)	3.70 (11.111)	0.00 (0.000)	11.11 (17.213)	0.00 (0.000)	6.67 (13.801)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 0.0	0.0, 33.3	0.0, 0.0	0.0, 33.3	0.0, 0.0
Week 88						
n	7	7	3	3	10	10
Mean (SD)	0.00 (0.000)	0.00 (0.000)	22.22 (19.245)	11.11 (19.245)	6.67 (14.055)	3.33 (10.541)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Appetite Loss

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	0.00 (0.000)	0.00 (0.000)	66.67 (NA)	33.33 (NA)	16.67 (33.333)	8.33 (16.667)
Median	0.00	0.00	66.67	33.33	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	33.3, 33.3	0.0, 66.7	0.0, 33.3

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Modified Randomized Set

Constipation

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		9		33	
Mean (SD)	16.67 (27.802)		14.81 (17.568)		16.16 (25.168)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 33.3		0.0, 100.0	
Week 2						
n	24	24	9	8		
Mean (SD)	13.89 (29.352)	-2.78 (19.453)	22.22 (33.333)	4.17 (33.034)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Constipation

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	24	24	10	9		
Mean (SD)	15.28 (27.766)	-1.39 (25.020)	20.00 (32.203)	7.41 (22.222)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	0.0, 66.7		
Week 8						
n	19	19	7	6		
Mean (SD)	8.77 (18.732)	-3.51 (18.904)	23.81 (31.706)	11.11 (17.213)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 66.7	0.0, 33.3		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Constipation

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	10	9		
Mean (SD)	16.67 (32.121)	-1.52 (26.181)	26.67 (34.427)	14.81 (24.216)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	0.0, 66.7		
Week 24						
n	21	21	10	9		
Mean (SD)	9.52 (26.125)	-7.94 (20.829)	20.00 (32.203)	7.41 (27.778)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Constipation

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	8	8	27	27
Mean (SD)	7.02 (17.843)	-12.28 (22.800)	29.17 (27.817)	16.67 (25.198)	13.58 (23.130)	-3.70 (26.688)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 66.7	0.0, 66.7	0.0, 66.7	-66.7, 66.7
Week 56						
n	13	13	7	6	20	19
Mean (SD)	20.51 (32.026)	-2.56 (21.350)	14.29 (17.817)	-5.56 (13.608)	18.33 (27.519)	-3.51 (18.904)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-33.3, 0.0	0.0, 100.0	-66.7, 33.3

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Constipation

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	6	5	15	14
Mean (SD)	25.93 (32.394)	0.00 (23.570)	5.56 (13.608)	-6.67 (14.907)	17.78 (27.794)	-2.38 (20.524)
Median	33.33	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 33.3	-33.3, 0.0	0.0, 100.0	-33.3, 33.3
Week 88						
n	7	7	3	3	10	10
Mean (SD)	19.05 (17.817)	0.00 (19.245)	33.33 (57.735)	22.22 (38.490)	23.33 (31.623)	6.67 (26.294)
Median	33.33	0.00	0.00	0.00	16.67	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	0.0, 66.7	0.0, 100.0	-33.3, 66.7

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Constipation

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	22.22 (19.245)	-11.11 (19.245)	66.67 (NA)	33.33 (NA)	33.33 (27.217)	0.00 (27.217)
Median	33.33	0.00	66.67	33.33	33.33	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	66.7, 66.7	33.3, 33.3	0.0, 66.7	-33.3, 33.3

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Modified Randomized Set

Diarrhea

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		9		33	
Mean (SD)	2.78 (9.411)		7.41 (14.699)		4.04 (11.048)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 33.3		0.0, 33.3		0.0, 33.3	
Week 2						
n	24	24	9	8		
Mean (SD)	2.78 (9.411)	0.00 (0.000)	3.70 (11.111)	-4.17 (21.362)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 0.0	0.0, 33.3	-33.3, 33.3		

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Modified Randomized Set

Diarrhea

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	24	24	10	9		
Mean (SD)	4.17 (11.261)	1.39 (11.955)	3.33 (10.541)	-3.70 (20.031)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3		
Week 8						
n	19	19	7	6		
Mean (SD)	8.77 (18.732)	5.26 (16.715)	4.76 (12.599)	-5.56 (25.092)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	0.0, 66.7	0.0, 33.3	-33.3, 33.3		

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Modified Randomized Set

Diarrhea

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	10	9		
Mean (SD)	9.09 (15.195)	6.06 (13.159)	3.33 (10.541)	-3.70 (20.031)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	-33.3, 33.3		
Week 24						
n	21	21	10	9		
Mean (SD)	4.76 (11.952)	3.17 (14.548)	6.67 (14.055)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	0.0, 0.0		

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Modified Randomized Set

Diarrhea

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	8	8	27	27
Mean (SD)	5.26 (12.488)	1.75 (13.488)	4.17 (11.785)	-4.17 (21.362)	4.94 (12.067)	0.00 (16.013)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3
Week 56						
n	13	13	7	6	20	19
Mean (SD)	7.69 (14.618)	7.69 (14.618)	9.52 (16.265)	-5.56 (25.092)	8.33 (14.809)	3.51 (18.904)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3

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Modified Randomized Set

Diarrhea

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	6	5	14	13
Mean (SD)	0.00 (0.000)	0.00 (0.000)	22.22 (40.369)	13.33 (50.553)	9.52 (27.514)	5.13 (29.957)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0
Week 88						
n	7	7	3	3	10	10
Mean (SD)	4.76 (12.599)	4.76 (12.599)	0.00 (0.000)	-11.11 (19.245)	3.33 (10.541)	0.00 (15.713)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 0.0	-33.3, 0.0	0.0, 33.3	-33.3, 33.3

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Diarrhea

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (NA)	0.00 (NA)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0

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Modified Randomized Set

Financial Difficulties

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		9		33	
Mean (SD)	20.83 (35.184)		33.33 (37.268)		24.24 (35.622)	
Median	0.00		33.33		0.00	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	24	24	9	8		
Mean (SD)	20.83 (36.531)	0.00 (13.901)	22.22 (33.333)	-8.33 (29.547)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 33.3		

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Financial Difficulties

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	24	24	10	9		
Mean (SD)	19.44 (32.477)	-1.39 (18.334)	16.67 (32.394)	-14.81 (29.397)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 33.3		
Week 8						
n	19	19	7	6		
Mean (SD)	12.28 (25.363)	-7.02 (21.020)	14.29 (17.817)	0.00 (21.082)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-33.3, 33.3		

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Modified Randomized Set

Financial Difficulties

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	10	9		
Mean (SD)	21.21 (34.953)	0.00 (14.548)	23.33 (35.312)	-7.41 (36.430)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 66.7		
Week 24						
n	21	21	10	9		
Mean (SD)	12.70 (30.689)	-7.94 (23.345)	16.67 (32.394)	-14.81 (24.216)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-100.0, 0.0	0.0, 100.0	-66.7, 0.0		

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Modified Randomized Set

Financial Difficulties

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	8	8	27	27
Mean (SD)	15.79 (30.161)	-8.77 (24.450)	16.67 (25.198)	-20.83 (39.591)	16.05 (28.300)	-12.35 (29.451)
Median	0.00	0.00	0.00	-16.67	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	-100.0, 33.3	0.0, 100.0	-100.0, 33.3
Week 56						
n	13	13	7	6	20	19
Mean (SD)	15.38 (32.247)	-10.26 (21.014)	23.81 (37.090)	-11.11 (27.217)	18.33 (33.289)	-10.53 (22.368)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 0.0	0.0, 100.0	-66.7, 0.0	0.0, 100.0	-66.7, 0.0

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Table GERMANY.2.5.4.5

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Modified Randomized Set

Financial Difficulties

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	6	5	14	13
Mean (SD)	4.17 (11.785)	-8.33 (15.430)	11.11 (17.213)	-13.33 (29.814)	7.14 (14.194)	-10.26 (21.014)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0
Week 88						
n	7	7	3	3	10	10
Mean (SD)	4.76 (12.599)	-9.52 (25.198)	11.11 (19.245)	-22.22 (38.490)	6.67 (14.055)	-13.33 (28.109)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0

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Financial Difficulties

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (NA)	-66.67 (NA)	0.00 (0.000)	-16.67 (33.333)
Median	0.00	0.00	0.00	-66.67	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	-66.7, -66.7	0.0, 0.0	-66.7, 0.0

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Fatigue

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		15		48	
Mean (SD)	42.09 (28.566)		44.44 (30.861)		42.82 (28.991)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	31	31	16	15		
Mean (SD)	31.54 (25.190)	-11.11 (23.307)	47.92 (24.248)	3.70 (24.003)		
Median	33.33	-11.11	38.89	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	11.1, 100.0	-44.4, 44.4		

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Modified Randomized Set

Fatigue

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	33	33	15	15		
Mean (SD)	27.78 (23.976)	-14.31 (26.463)	41.48 (17.042)	-2.96 (27.043)		
Median	22.22	-11.11	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 44.4	11.1, 66.7	-66.7, 33.3		
Week 8						
n	26	26	12	12		
Mean (SD)	26.92 (16.974)	-15.38 (23.323)	37.96 (20.899)	-1.85 (28.361)		
Median	33.33	-16.67	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 22.2	0.0, 77.8	-77.8, 44.4		

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Fatigue

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	15	15		
Mean (SD)	27.04 (20.149)	-15.93 (21.969)	49.63 (31.669)	5.19 (37.058)		
Median	27.78	-11.11	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 22.2	11.1, 100.0	-77.8, 77.8		
Week 24						
n	28	28	14	14		
Mean (SD)	25.40 (21.566)	-17.06 (27.630)	28.57 (26.407)	-11.90 (35.262)		
Median	22.22	-11.11	22.22	-16.67		
Min, Max	0.0, 100.0	-77.8, 33.3	0.0, 88.9	-55.6, 77.8		

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Fatigue

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	13	13	40	40
Mean (SD)	25.10 (21.482)	-17.70 (24.510)	33.33 (29.745)	-8.55 (33.381)	27.78 (24.395)	-14.72 (27.608)
Median	22.22	-11.11	33.33	0.00	22.22	-11.11
Min, Max	0.0, 100.0	-77.8, 33.3	0.0, 100.0	-55.6, 66.7	0.0, 100.0	-77.8, 66.7
Week 56						
n	21	21	10	10	31	31
Mean (SD)	30.69 (20.459)	-12.17 (26.505)	31.11 (14.628)	-11.11 (33.945)	30.82 (18.528)	-11.83 (28.536)
Median	33.33	0.00	27.78	-11.11	33.33	-11.11
Min, Max	0.0, 100.0	-66.7, 33.3	11.1, 55.6	-66.7, 44.4	0.0, 100.0	-66.7, 44.4

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Modified Randomized Set

Fatigue

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	6	6	18	18
Mean (SD)	21.30 (18.015)	-24.07 (23.130)	57.41 (27.592)	5.56 (35.660)	33.33 (27.217)	-14.20 (30.442)
Median	22.22	-11.11	61.11	0.00	33.33	-11.11
Min, Max	0.0, 55.6	-66.7, 0.0	22.2, 100.0	-33.3, 66.7	0.0, 100.0	-66.7, 66.7
Week 88						
n	7	7	5	5	12	12
Mean (SD)	26.98 (20.141)	-15.87 (31.333)	44.44 (29.397)	-11.11 (42.310)	34.26 (24.826)	-13.89 (34.532)
Median	22.22	-22.22	55.56	-11.11	33.33	-16.67
Min, Max	11.1, 66.7	-66.7, 33.3	0.0, 77.8	-66.7, 44.4	0.0, 77.8	-66.7, 44.4

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Modified Randomized Set

Fatigue

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	-33.33 (NA)	22.22 (NA)	-77.78 (NA)	11.11 (15.713)	-55.56 (31.427)
Median	0.00	-33.33	22.22	-77.78	11.11	-55.56
Min, Max	0.0, 0.0	-33.3, -33.3	22.2, 22.2	-77.8, -77.8	0.0, 22.2	-77.8, -33.3

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Nausea and Vomiting

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		15		48	
Mean (SD)	4.55 (13.355)		7.78 (18.758)		5.56 (15.117)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	31	31	16	15		
Mean (SD)	3.76 (10.283)	-1.08 (14.867)	8.33 (16.102)	-1.11 (7.629)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 50.0	-50.0, 50.0	0.0, 50.0	-16.7, 16.7		

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Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	33	33	15	15		
Mean (SD)	4.55 (10.436)	0.00 (14.434)	2.22 (8.607)	-5.56 (19.586)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-50.0, 33.3	0.0, 33.3	-66.7, 16.7		
Week 8						
n	26	26	12	12		
Mean (SD)	3.85 (9.784)	-1.92 (11.857)	6.94 (15.006)	0.00 (23.570)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 50.0	-66.7, 33.3		

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	15	15		
Mean (SD)	3.89 (9.472)	-0.56 (11.973)	8.89 (21.700)	1.11 (23.117)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-50.0, 33.3	0.0, 83.3	-66.7, 50.0		
Week 24						
n	28	28	14	14		
Mean (SD)	4.17 (13.322)	-0.60 (17.262)	3.57 (7.097)	0.00 (13.074)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-50.0, 66.7	0.0, 16.7	-33.3, 16.7		

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Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	13	13	40	40
Mean (SD)	4.94 (7.755)	2.47 (7.601)	6.41 (14.495)	2.56 (16.452)	5.42 (10.258)	2.50 (11.036)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	-16.7, 16.7	0.0, 50.0	-16.7, 50.0	0.0, 50.0	-16.7, 50.0
Week 56						
n	21	21	10	10	31	31
Mean (SD)	0.79 (3.637)	-2.38 (9.554)	3.33 (7.027)	-1.67 (12.298)	1.61 (5.009)	-2.15 (10.312)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	-33.3, 16.7	0.0, 16.7	-33.3, 16.7	0.0, 16.7	-33.3, 16.7

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Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	6	6	18	18
Mean (SD)	0.00 (0.000)	-2.78 (6.487)	11.11 (13.608)	2.78 (24.533)	3.70 (9.139)	-0.93 (14.543)
Median	0.00	0.00	8.33	8.33	0.00	0.00
Min, Max	0.0, 0.0	-16.7, 0.0	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3
Week 88						
n	7	7	5	5	12	12
Mean (SD)	2.38 (6.299)	0.00 (0.000)	6.67 (9.129)	-3.33 (18.257)	4.17 (7.538)	-1.39 (11.143)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	0.0, 0.0	0.0, 16.7	-33.3, 16.7	0.0, 16.7	-33.3, 16.7

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Nausea and Vomiting

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	66.67 (NA)	33.33 (NA)	33.33 (47.140)	16.67 (23.570)
Median	0.00	0.00	66.67	33.33	33.33	16.67
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	33.3, 33.3	0.0, 66.7	0.0, 33.3

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Modified Randomized Set

Pain

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		15		48	
Mean (SD)	17.68 (24.274)		20.00 (16.903)		18.40 (22.079)	
Median	0.00		16.67		16.67	
Min, Max	0.0, 83.3		0.0, 50.0		0.0, 83.3	
Week 2						
n	31	31	15	14		
Mean (SD)	12.37 (18.742)	-6.45 (17.574)	23.33 (18.687)	1.19 (20.111)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 50.0	-33.3, 33.3		

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Pain

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	33	33	15	15		
Mean (SD)	13.64 (18.378)	-4.04 (18.648)	23.33 (26.577)	3.33 (26.874)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 50.0	-66.7, 33.3	0.0, 100.0	-33.3, 83.3		
Week 8						
n	25	25	11	11		
Mean (SD)	12.67 (23.214)	-4.00 (25.129)	10.61 (13.484)	-9.09 (20.226)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 33.3	-33.3, 16.7		

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Modified Randomized Set

Pain

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	14	14		
Mean (SD)	12.22 (21.412)	-5.00 (24.428)	25.00 (31.180)	4.76 (35.463)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 66.7	-83.3, 33.3	0.0, 100.0	-33.3, 83.3		
Week 24						
n	28	28	14	14		
Mean (SD)	11.31 (23.153)	-5.95 (27.672)	14.29 (17.118)	-4.76 (18.979)		
Median	0.00	0.00	8.33	0.00		
Min, Max	0.0, 100.0	-83.3, 66.7	0.0, 50.0	-33.3, 33.3		

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Pain

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	13	13	40	40
Mean (SD)	10.49 (20.227)	-6.79 (23.687)	19.23 (27.927)	1.28 (29.235)	13.33 (23.020)	-4.17 (25.529)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 66.7	-83.3, 33.3	0.0, 100.0	-33.3, 83.3	0.0, 100.0	-83.3, 83.3
Week 56						
n	21	21	10	10	31	31
Mean (SD)	12.70 (21.670)	-7.94 (25.614)	21.67 (17.656)	3.33 (24.595)	15.59 (20.609)	-4.30 (25.447)
Median	0.00	0.00	25.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-83.3, 33.3	0.0, 50.0	-33.3, 50.0	0.0, 66.7	-83.3, 50.0

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Pain

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	6	6	18	18
Mean (SD)	6.94 (11.143)	-13.89 (22.285)	30.56 (40.023)	11.11 (45.542)	14.81 (26.127)	-5.56 (32.839)
Median	0.00	0.00	16.67	-8.33	0.00	0.00
Min, Max	0.0, 33.3	-50.0, 16.7	0.0, 100.0	-33.3, 83.3	0.0, 100.0	-50.0, 83.3
Week 88						
n	7	7	5	5	12	12
Mean (SD)	9.52 (13.113)	-11.90 (26.726)	26.67 (34.561)	13.33 (38.006)	16.67 (24.618)	-1.39 (32.920)
Median	0.00	0.00	16.67	0.00	8.33	0.00
Min, Max	0.0, 33.3	-66.7, 16.7	0.0, 83.3	-33.3, 66.7	0.0, 83.3	-66.7, 66.7

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Modified Randomized Set

Pain

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	16.67 (NA)	0.00 (NA)	8.33 (11.785)	0.00 (0.000)
Median	0.00	0.00	16.67	0.00	8.33	0.00
Min, Max	0.0, 0.0	0.0, 0.0	16.7, 16.7	0.0, 0.0	0.0, 16.7	0.0, 0.0

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Dyspnoea

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		15		48	
Mean (SD)	37.37 (28.574)		31.11 (32.038)		35.42 (29.500)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	32	32	16	15		
Mean (SD)	21.88 (24.843)	-16.67 (20.739)	39.58 (30.353)	11.11 (27.217)		
Median	16.67	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 100.0	-33.3, 66.7		

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Dyspnoea

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	33	33	15	15		
Mean (SD)	12.12 (20.101)	-25.25 (23.615)	40.00 (38.214)	8.89 (34.427)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 100.0	-66.7, 66.7		
Week 8						
n	26	26	12	12		
Mean (SD)	12.82 (19.037)	-24.36 (22.226)	33.33 (31.782)	-2.78 (41.337)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 66.7	-100.0, 0.0	0.0, 100.0	-100.0, 66.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Dyspnoea

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	15	15		
Mean (SD)	16.67 (22.743)	-20.00 (24.132)	28.89 (27.794)	-2.22 (44.484)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-100.0, 66.7		
Week 24						
n	28	28	14	14		
Mean (SD)	15.48 (23.098)	-17.86 (29.372)	14.29 (17.118)	-11.90 (28.063)		
Median	0.00	-33.33	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 33.3	-66.7, 33.3		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Dyspnoea

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	13	13	40	40
Mean (SD)	13.58 (19.080)	-25.93 (21.350)	15.38 (17.296)	-10.26 (25.036)	14.17 (18.316)	-20.83 (23.495)
Median	0.00	-33.33	0.00	0.00	0.00	-33.33
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-66.7, 33.3
Week 56						
n	21	21	10	10	31	31
Mean (SD)	19.05 (24.881)	-19.05 (24.881)	16.67 (17.568)	0.00 (22.222)	18.28 (22.507)	-12.90 (25.353)
Median	0.00	-33.33	16.67	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-66.7, 33.3

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Modified Randomized Set

Dyspnoea

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	6	6	18	18
Mean (SD)	11.11 (21.711)	-36.11 (17.164)	16.67 (27.889)	-16.67 (34.960)	12.96 (23.260)	-29.63 (25.280)
Median	0.00	-33.33	0.00	-16.67	0.00	-33.33
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-66.7, 33.3
Week 88						
n	7	7	5	5	12	12
Mean (SD)	14.29 (26.227)	-28.57 (23.002)	6.67 (14.907)	-26.67 (27.889)	11.11 (21.711)	-27.78 (23.925)
Median	0.00	-33.33	0.00	-33.33	0.00	-33.33
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 66.7	-66.7, 0.0

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Table GERMANY.2.5.4.5

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Modified Randomized Set

Dyspnoea

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	-33.33 (NA)	33.33 (NA)	0.00 (NA)	16.67 (23.570)	-16.67 (23.570)
Median	0.00	-33.33	33.33	0.00	16.67	-16.67
Min, Max	0.0, 0.0	-33.3, -33.3	33.3, 33.3	0.0, 0.0	0.0, 33.3	-33.3, 0.0

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Modified Randomized Set

Insomnia

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		15		48	
Mean (SD)	30.30 (28.092)		33.33 (37.796)		31.25 (31.062)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	31	31	16	15		
Mean (SD)	22.58 (27.738)	-8.60 (17.144)	33.33 (32.203)	0.00 (33.333)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 66.7		

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Insomnia

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	33	33	15	15		
Mean (SD)	21.21 (33.143)	-9.09 (27.979)	24.44 (29.457)	-8.89 (36.659)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-100.0, 33.3		
Week 8						
n	26	26	12	12		
Mean (SD)	11.54 (18.720)	-19.23 (23.425)	22.22 (29.588)	-5.56 (37.155)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 100.0	-100.0, 33.3		

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Modified Randomized Set

Insomnia

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	15	15		
Mean (SD)	16.67 (24.369)	-13.33 (20.715)	35.56 (32.038)	2.22 (36.659)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 0.0	0.0, 100.0	-66.7, 66.7		
Week 24						
n	28	28	14	14		
Mean (SD)	15.48 (27.936)	-14.29 (29.297)	21.43 (28.063)	-11.90 (28.063)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 66.7	-66.7, 33.3		

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Insomnia

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	13	13	40	40
Mean (SD)	18.52 (28.244)	-12.35 (24.717)	17.95 (22.008)	-17.95 (37.553)	18.33 (26.094)	-14.17 (29.125)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	-100.0, 33.3	0.0, 100.0	-100.0, 33.3
Week 56						
n	21	21	10	10	31	31
Mean (SD)	25.40 (29.636)	-7.94 (23.345)	36.67 (33.148)	-6.67 (34.427)	29.03 (30.722)	-7.53 (26.819)
Median	33.33	0.00	33.33	0.00	33.33	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 33.3

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Insomnia

≤ 2 Transfusions During 6 Months Prior to Screening

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	6	6	18	18
Mean (SD)	11.11 (16.412)	-19.44 (22.285)	44.44 (40.369)	-16.67 (62.361)	22.22 (30.250)	-18.52 (38.301)
Median	0.00	-16.67	50.00	-16.67	0.00	-16.67
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 100.0	-100.0, 66.7	0.0, 100.0	-100.0, 66.7
Week 88						
n	7	7	5	5	12	12
Mean (SD)	14.29 (26.227)	-14.29 (17.817)	40.00 (43.461)	-13.33 (50.553)	25.00 (35.176)	-13.89 (33.207)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 33.3

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	33.33 (NA)	0.00 (NA)	0.00 (NA)	-100.00 (NA)	16.67 (23.570)	-50.00 (70.711)
Median	33.33	0.00	0.00	-100.00	16.67	-50.00
Min, Max	33.3, 33.3	0.0, 0.0	0.0, 0.0	-100.0, -100.0	0.0, 33.3	-100.0, 0.0

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Appetite Loss

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		15		48	
Mean (SD)	8.08 (14.506)		17.78 (30.516)		11.11 (21.007)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 33.3		0.0, 100.0		0.0, 100.0	
Week 2						
n	32	32	16	15		
Mean (SD)	7.29 (14.000)	-1.04 (15.804)	20.83 (26.874)	2.22 (15.258)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-33.3, 33.3		

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Modified Randomized Set

Appetite Loss

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	33	33	15	15		
Mean (SD)	5.05 (12.137)	-3.03 (12.812)	13.33 (30.342)	-4.44 (33.014)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-66.7, 66.7		
Week 8						
n	26	26	12	12		
Mean (SD)	12.82 (19.037)	2.56 (22.945)	11.11 (21.711)	-2.78 (33.207)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-100.0, 33.3		

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Appetite Loss

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	15	15		
Mean (SD)	4.44 (14.472)	-2.22 (21.323)	17.78 (30.516)	0.00 (25.198)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 100.0	-66.7, 33.3		
Week 24						
n	28	28	14	14		
Mean (SD)	10.71 (22.321)	3.57 (26.198)	16.67 (21.681)	4.76 (12.105)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 66.7	0.0, 33.3		

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Appetite Loss

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	13	13	40	40
Mean (SD)	6.17 (13.195)	-2.47 (15.814)	17.95 (29.235)	5.13 (32.903)	10.00 (20.255)	0.00 (22.646)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0
Week 56						
n	21	21	10	10	31	31
Mean (SD)	7.94 (14.548)	-1.59 (22.301)	13.33 (32.203)	-3.33 (33.148)	9.68 (21.419)	-2.15 (25.727)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Appetite Loss

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	6	6	18	18
Mean (SD)	2.78 (9.623)	-8.33 (15.076)	27.78 (25.092)	5.56 (32.773)	11.11 (19.803)	-3.70 (22.547)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-33.3, 66.7
Week 88						
n	7	7	5	5	12	12
Mean (SD)	14.29 (17.817)	0.00 (19.245)	13.33 (18.257)	-6.67 (36.515)	13.89 (17.164)	-2.78 (26.432)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Appetite Loss

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	66.67 (NA)	0.00 (NA)	33.33 (47.140)	0.00 (0.000)
Median	0.00	0.00	66.67	0.00	33.33	0.00
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	0.0, 0.0	0.0, 66.7	0.0, 0.0

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Constipation

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		15		48	
Mean (SD)	11.11 (19.837)		8.89 (19.787)		10.42 (19.638)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	31	31	16	15		
Mean (SD)	6.45 (15.915)	-5.38 (19.430)	14.58 (29.736)	6.67 (22.537)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

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Constipation

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	33	33	15	15		
Mean (SD)	6.06 (17.588)	-5.05 (18.858)	17.78 (27.794)	8.89 (26.627)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		
Week 8						
n	26	26	12	12		
Mean (SD)	3.85 (10.860)	-8.97 (22.226)	11.11 (29.588)	5.56 (12.975)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 100.0	0.0, 33.3		

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Modified Randomized Set

Constipation

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	15	15		
Mean (SD)	4.44 (14.472)	-5.56 (15.371)	11.11 (29.991)	2.22 (19.787)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-33.3, 66.7		
Week 24						
n	28	28	14	14		
Mean (SD)	4.76 (11.878)	-4.76 (19.698)	14.29 (21.540)	4.76 (17.817)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-33.3, 33.3		

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Constipation

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	13	13	40	40
Mean (SD)	6.17 (13.195)	-6.17 (20.749)	7.69 (14.618)	-2.56 (16.452)	6.67 (13.503)	-5.00 (19.319)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3
Week 56						
n	21	21	10	10	31	31
Mean (SD)	3.17 (10.026)	-11.11 (19.245)	10.00 (22.498)	3.33 (10.541)	5.38 (15.146)	-6.45 (18.093)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 66.7	0.0, 33.3	0.0, 66.7	-66.7, 33.3

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Constipation

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	6	6	18	18
Mean (SD)	2.78 (9.623)	-13.89 (17.164)	16.67 (18.257)	11.11 (17.213)	7.41 (14.260)	-5.56 (20.612)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	0.0, 33.3	0.0, 33.3	-33.3, 33.3
Week 88						
n	7	7	5	5	12	12
Mean (SD)	9.52 (16.265)	-9.52 (25.198)	20.00 (44.721)	13.33 (50.553)	13.89 (30.011)	0.00 (37.605)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0

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Constipation

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	0.00 (NA)	-33.33 (NA)	0.00 (0.000)	-16.67 (23.570)
Median	0.00	0.00	0.00	-33.33	0.00	-16.67
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	-33.3, -33.3	0.0, 0.0	-33.3, 0.0

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Diarrhea

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		15		48	
Mean (SD)	7.07 (19.996)		15.56 (24.774)		9.72 (21.699)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	32	32	16	15		
Mean (SD)	8.33 (22.401)	1.04 (23.163)	6.25 (18.130)	-8.89 (23.458)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 100.0	0.0, 66.7	-66.7, 33.3		

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Modified Randomized Set

Diarrhea

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	33	33	15	15		
Mean (SD)	6.06 (15.489)	-1.01 (24.274)	4.44 (11.729)	-11.11 (20.574)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-100.0, 66.7	0.0, 33.3	-66.7, 0.0		
Week 8						
n	26	26	12	12		
Mean (SD)	6.41 (13.397)	0.00 (21.082)	8.33 (20.719)	-8.33 (15.076)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-33.3, 0.0		

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Modified Randomized Set

Diarrhea

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	15	15		
Mean (SD)	3.33 (10.171)	-4.44 (16.914)	4.44 (17.213)	-11.11 (32.530)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-66.7, 66.7		
Week 24						
n	28	28	14	14		
Mean (SD)	5.95 (13.001)	-1.19 (16.929)	2.38 (8.909)	-11.90 (21.111)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 0.0		

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Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	13	13	40	40
Mean (SD)	4.94 (12.067)	-3.70 (25.036)	10.26 (16.013)	-5.13 (26.688)	6.67 (13.503)	-4.17 (25.248)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-100.0, 33.3
Week 56						
n	21	21	10	10	31	31
Mean (SD)	6.35 (13.412)	-1.59 (24.667)	6.67 (14.055)	-13.33 (23.307)	6.45 (13.387)	-5.38 (24.490)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-100.0, 33.3

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Diarrhea

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	6	6	18	18
Mean (SD)	0.00 (0.000)	-2.78 (9.623)	27.78 (25.092)	0.00 (21.082)	9.26 (19.150)	-1.85 (13.873)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3
Week 88						
n	7	7	5	5	12	12
Mean (SD)	0.00 (0.000)	0.00 (0.000)	13.33 (18.257)	-6.67 (36.515)	5.56 (12.975)	-2.78 (22.285)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	0.00 (NA)	0.00 (NA)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Financial Difficulties

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		15		48	
Mean (SD)	20.20 (27.562)		17.78 (27.794)		19.44 (27.361)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	33	33	16	15		
Mean (SD)	16.16 (23.748)	-4.04 (16.154)	25.00 (35.486)	8.89 (26.627)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 100.0	0.0, 100.0		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Modified Randomized Set

Financial Difficulties

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	33	33	15	15		
Mean (SD)	16.16 (22.238)	-4.04 (21.663)	20.00 (35.187)	2.22 (29.457)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 66.7	0.0, 100.0	-33.3, 100.0		
Week 8						
n	26	26	12	12		
Mean (SD)	12.82 (19.037)	-6.41 (23.131)	13.89 (26.432)	0.00 (20.101)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-100.0, 33.3	0.0, 66.7	-33.3, 33.3		

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Financial Difficulties

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	15	15		
Mean (SD)	15.56 (19.045)	-4.44 (19.045)	22.22 (37.090)	4.44 (30.516)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-33.3, 100.0		
Week 24						
n	28	28	14	14		
Mean (SD)	21.43 (27.539)	1.19 (26.422)	26.19 (37.390)	7.14 (29.753)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 100.0	0.0, 100.0	-33.3, 100.0		

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Financial Difficulties

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	13	13	40	40
Mean (SD)	18.52 (25.036)	-2.47 (22.505)	23.08 (36.980)	2.56 (31.802)	20.00 (29.040)	-0.83 (25.584)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-66.7, 100.0
Week 56						
n	21	21	10	10	31	31
Mean (SD)	11.11 (16.102)	-7.94 (17.965)	26.67 (34.427)	3.33 (45.677)	16.13 (24.146)	-4.30 (29.492)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-66.7, 100.0	0.0, 100.0	-66.7, 100.0

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Table GERMANY.2.5.4.5

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Modified Randomized Set

Financial Difficulties

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	6	6	18	18
Mean (SD)	11.11 (16.412)	-11.11 (29.588)	11.11 (27.217)	-16.67 (18.257)	11.11 (19.803)	-12.96 (25.918)
Median	0.00	0.00	0.00	-16.67	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-33.3, 0.0	0.0, 66.7	-66.7, 33.3
Week 88						
n	7	7	5	5	12	12
Mean (SD)	14.29 (17.817)	-9.52 (16.265)	6.67 (14.907)	-26.67 (27.889)	11.11 (16.412)	-16.67 (22.473)
Median	0.00	0.00	0.00	-33.33	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0

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Table GERMANY.2.5.4.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Financial Difficulties

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	-66.67 (NA)	33.33 (NA)	-66.67 (NA)	16.67 (23.570)	-66.67 (0.000)
Median	0.00	-66.67	33.33	-66.67	16.67	-66.67
Min, Max	0.0, 0.0	-66.7, -66.7	33.3, 33.3	-66.7, -66.7	0.0, 33.3	-66.7, -66.7

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Fatigue

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		15		50	
Mean (SD)	46.35 (23.413)		47.41 (27.043)		46.67 (24.281)	
Median	33.33		44.44		38.89	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	33	33	15	15		
Mean (SD)	33.33 (21.337)	-13.80 (22.572)	42.96 (17.247)	-4.44 (20.914)		
Median	33.33	-11.11	33.33	0.00		
Min, Max	0.0, 88.9	-66.7, 33.3	11.1, 66.7	-44.4, 33.3		

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Modified Randomized Set

Fatigue

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	35	35	15	15		
Mean (SD)	29.37 (18.747)	-16.98 (22.504)	50.37 (24.798)	2.96 (24.295)		
Median	33.33	-11.11	33.33	0.00		
Min, Max	0.0, 77.8	-66.7, 22.2	11.1, 100.0	-66.7, 44.4		
Week 8						
n	29	29	12	12		
Mean (SD)	36.40 (22.595)	-9.58 (21.357)	37.04 (22.886)	-8.33 (30.750)		
Median	33.33	-11.11	33.33	0.00		
Min, Max	0.0, 100.0	-44.4, 44.4	0.0, 77.8	-77.8, 44.4		

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Modified Randomized Set

Fatigue

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	32	32	15	15		
Mean (SD)	30.21 (18.981)	-15.63 (20.712)	44.44 (29.397)	-2.96 (33.192)		
Median	33.33	-11.11	44.44	0.00		
Min, Max	0.0, 66.7	-66.7, 22.2	0.0, 100.0	-77.8, 66.7		
Week 24						
n	29	29	14	14		
Mean (SD)	24.90 (20.925)	-20.69 (24.796)	32.54 (31.265)	-11.11 (27.906)		
Median	22.22	-22.22	27.78	-5.56		
Min, Max	0.0, 88.9	-77.8, 33.3	0.0, 100.0	-55.6, 44.4		

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Modified Randomized Set

Fatigue

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	12	12	41	41
Mean (SD)	25.67 (21.228)	-17.24 (22.923)	40.74 (35.560)	-0.93 (32.981)	30.08 (26.672)	-12.47 (26.897)
Median	22.22	-11.11	33.33	0.00	22.22	-11.11
Min, Max	0.0, 77.8	-77.8, 11.1	0.0, 100.0	-44.4, 66.7	0.0, 100.0	-77.8, 66.7
Week 56						
n	21	21	10	10	31	31
Mean (SD)	30.16 (19.920)	-11.11 (23.831)	31.11 (14.628)	-11.11 (22.831)	30.47 (18.137)	-11.11 (23.130)
Median	33.33	-11.11	33.33	-11.11	33.33	-11.11
Min, Max	0.0, 66.7	-66.7, 33.3	11.1, 66.7	-55.6, 33.3	0.0, 66.7	-66.7, 33.3

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Table GERMANY.2.5.4.6
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Fatigue

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	7	7	19	19
Mean (SD)	25.93 (16.639)	-22.22 (23.210)	47.62 (27.000)	4.76 (28.586)	33.92 (22.973)	-12.28 (27.937)
Median	27.78	-11.11	33.33	0.00	33.33	-11.11
Min, Max	0.0, 55.6	-66.7, 0.0	22.2, 100.0	-22.2, 66.7	0.0, 100.0	-66.7, 66.7
Week 88						
n	8	8	5	5	13	13
Mean (SD)	15.28 (13.197)	-26.39 (22.173)	42.22 (28.760)	0.00 (30.429)	25.64 (23.738)	-16.24 (27.820)
Median	11.11	-22.22	44.44	0.00	22.22	-22.22
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 77.8	-33.3, 44.4	0.0, 77.8	-66.7, 44.4

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Modified Randomized Set

Fatigue

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	11.11 (15.713)	-22.22 (15.713)	33.33 (NA)	0.00 (NA)	18.52 (16.973)	-14.81 (16.973)
Median	11.11	-22.22	33.33	0.00	22.22	-11.11
Min, Max	0.0, 22.2	-33.3, -11.1	33.3, 33.3	0.0, 0.0	0.0, 33.3	-33.3, 0.0

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Nausea and Vomiting

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		15		50	
Mean (SD)	1.90 (6.729)		6.67 (17.593)		3.33 (11.168)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 33.3		0.0, 66.7		0.0, 66.7	
Week 2						
n	33	33	15	15		
Mean (SD)	3.54 (9.998)	1.52 (12.050)	6.67 (15.171)	0.00 (8.909)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 50.0	-33.3, 50.0	0.0, 50.0	-16.7, 16.7		

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Modified Randomized Set

Nausea and Vomiting

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	35	35	15	15		
Mean (SD)	3.81 (9.972)	1.90 (11.269)	3.33 (6.901)	-3.33 (18.039)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 16.7	-66.7, 16.7		
Week 8						
n	29	29	12	12		
Mean (SD)	5.17 (16.728)	2.87 (18.403)	4.17 (7.538)	-2.78 (21.122)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 83.3	-33.3, 83.3	0.0, 16.7	-66.7, 16.7		

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Modified Randomized Set

Nausea and Vomiting

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	32	32	15	15		
Mean (SD)	3.13 (8.918)	1.04 (7.256)	2.22 (5.864)	-4.44 (19.382)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-16.7, 33.3	0.0, 16.7	-66.7, 16.7		
Week 24						
n	29	29	14	14		
Mean (SD)	1.72 (6.821)	-0.57 (8.313)	2.38 (6.052)	0.00 (9.245)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-16.7, 33.3	0.0, 16.7	-16.7, 16.7		

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Nausea and Vomiting

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	12	12	41	41
Mean (SD)	4.60 (9.857)	2.87 (10.029)	5.56 (14.794)	4.17 (16.088)	4.88 (11.331)	3.25 (11.914)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-16.7, 33.3	0.0, 50.0	-16.7, 50.0	0.0, 50.0	-16.7, 50.0
Week 56						
n	21	21	10	10	31	31
Mean (SD)	0.00 (0.000)	-2.38 (7.968)	13.33 (31.230)	11.67 (26.117)	4.30 (18.241)	2.15 (17.074)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 100.0	0.0, 83.3	0.0, 100.0	-33.3, 83.3

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Modified Randomized Set

Nausea and Vomiting

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	7	7	19	19
Mean (SD)	0.00 (0.000)	-1.39 (4.811)	7.14 (13.113)	7.14 (13.113)	2.63 (8.358)	1.75 (9.452)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	-16.7, 0.0	0.0, 33.3	0.0, 33.3	0.0, 33.3	-16.7, 33.3
Week 88						
n	8	8	5	5	13	13
Mean (SD)	0.00 (0.000)	0.00 (0.000)	6.67 (9.129)	6.67 (9.129)	2.56 (6.259)	2.56 (6.259)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 16.7	0.0, 16.7	0.0, 16.7	0.0, 16.7

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Modified Randomized Set

Nausea and Vomiting

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	0.00 (0.000)	0.00 (0.000)	33.33 (NA)	33.33 (NA)	11.11 (19.245)	11.11 (19.245)
Median	0.00	0.00	33.33	33.33	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	33.3, 33.3	33.3, 33.3	0.0, 33.3	0.0, 33.3

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Modified Randomized Set

Pain

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		15		50	
Mean (SD)	12.86 (24.617)		25.56 (28.776)		16.67 (26.298)	
Median	0.00		33.33		0.00	
Min, Max	0.0, 83.3		0.0, 100.0		0.0, 100.0	
Week 2						
n	33	33	14	14		
Mean (SD)	12.12 (22.543)	-1.52 (19.704)	23.81 (23.310)	-2.38 (17.118)		
Median	0.00	0.00	25.00	0.00		
Min, Max	0.0, 83.3	-66.7, 66.7	0.0, 66.7	-33.3, 33.3		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:28

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Pain

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	35	35	15	15		
Mean (SD)	10.00 (18.612)	-2.86 (17.844)	20.00 (26.874)	-5.56 (16.265)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 50.0	0.0, 83.3	-33.3, 16.7		
Week 8						
n	28	28	11	11		
Mean (SD)	14.88 (28.810)	5.95 (30.501)	9.09 (13.670)	-12.12 (18.395)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 33.3	-33.3, 16.7		

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Modified Randomized Set

Pain

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	32	32	14	14		
Mean (SD)	8.33 (16.933)	-5.21 (22.969)	17.86 (24.862)	-8.33 (19.337)		
Median	0.00	0.00	8.33	-8.33		
Min, Max	0.0, 66.7	-83.3, 33.3	0.0, 83.3	-33.3, 33.3		
Week 24						
n	29	29	14	14		
Mean (SD)	5.75 (11.159)	-8.05 (24.649)	13.10 (23.732)	-11.90 (23.956)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-83.3, 33.3	0.0, 83.3	-66.7, 16.7		

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Modified Randomized Set

Pain

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	12	12	41	41
Mean (SD)	9.20 (20.210)	-3.45 (22.440)	23.61 (37.240)	2.78 (30.011)	13.41 (26.672)	-1.63 (24.666)
Median	0.00	0.00	8.33	0.00	0.00	0.00
Min, Max	0.0, 83.3	-83.3, 33.3	0.0, 100.0	-33.3, 83.3	0.0, 100.0	-83.3, 83.3
Week 56						
n	21	21	10	10	31	31
Mean (SD)	11.90 (19.821)	-3.97 (30.689)	26.67 (23.831)	3.33 (26.989)	16.67 (21.943)	-1.61 (29.299)
Median	0.00	0.00	25.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-83.3, 66.7	0.0, 66.7	-33.3, 50.0	0.0, 66.7	-83.3, 66.7

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Pain

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	7	7	19	19
Mean (SD)	6.94 (11.143)	-9.72 (22.983)	26.19 (38.318)	14.29 (39.002)	14.04 (25.618)	-0.88 (31.167)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-50.0, 16.7	0.0, 100.0	-33.3, 83.3	0.0, 100.0	-50.0, 83.3
Week 88						
n	8	8	5	5	13	13
Mean (SD)	4.17 (7.715)	-8.33 (25.198)	26.67 (34.561)	16.67 (37.268)	12.82 (23.721)	1.28 (31.521)
Median	0.00	0.00	16.67	16.67	0.00	0.00
Min, Max	0.0, 16.7	-66.7, 16.7	0.0, 83.3	-33.3, 66.7	0.0, 83.3	-66.7, 66.7

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Pain

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	0.00 (0.000)	0.00 (0.000)	16.67 (NA)	16.67 (NA)	5.56 (9.623)	5.56 (9.623)
Median	0.00	0.00	16.67	16.67	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	16.7, 16.7	16.7, 16.7	0.0, 16.7	0.0, 16.7

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Dyspnoea

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		15		50	
Mean (SD)	39.05 (29.689)		33.33 (30.861)		37.33 (29.845)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	34	34	15	15		
Mean (SD)	25.49 (28.496)	-14.71 (24.878)	28.89 (21.331)	-4.44 (24.774)		
Median	33.33	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	-33.3, 33.3		

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Modified Randomized Set

Dyspnoea

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	35	35	15	15		
Mean (SD)	19.05 (25.928)	-20.00 (27.056)	33.33 (28.172)	0.00 (37.796)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 66.7		
Week 8						
n	29	29	12	12		
Mean (SD)	25.29 (24.649)	-11.49 (22.318)	30.56 (30.011)	-5.56 (42.243)		
Median	33.33	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-100.0, 66.7		

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Modified Randomized Set

Dyspnoea

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	32	32	15	15		
Mean (SD)	19.79 (20.495)	-18.75 (23.854)	31.11 (23.458)	-2.22 (44.484)		
Median	33.33	-16.67	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-100.0, 66.7		
Week 24						
n	29	29	14	14		
Mean (SD)	16.09 (21.121)	-20.69 (24.257)	14.29 (17.118)	-14.29 (25.198)		
Median	0.00	-33.33	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 33.3	-66.7, 33.3		

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Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	12	12	41	41
Mean (SD)	17.24 (24.593)	-20.69 (27.334)	13.89 (17.164)	-16.67 (22.473)	16.26 (22.512)	-19.51 (25.794)
Median	0.00	-33.33	0.00	0.00	0.00	-33.33
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-66.7, 0.0	0.0, 100.0	-66.7, 33.3
Week 56						
n	21	21	10	10	31	31
Mean (SD)	17.46 (24.987)	-20.63 (24.667)	13.33 (17.213)	-13.33 (28.109)	16.13 (22.560)	-18.28 (25.587)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-66.7, 33.3

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Dyspnoea

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	7	7	19	19
Mean (SD)	13.89 (22.285)	-33.33 (20.101)	23.81 (31.706)	-9.52 (41.786)	17.54 (25.744)	-24.56 (31.115)
Median	0.00	-33.33	0.00	-33.33	0.00	-33.33
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-66.7, 33.3
Week 88						
n	8	8	5	5	13	13
Mean (SD)	4.17 (11.785)	-33.33 (17.817)	13.33 (18.257)	-20.00 (38.006)	7.69 (14.618)	-28.21 (26.688)
Median	0.00	-33.33	0.00	-33.33	0.00	-33.33
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3

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Table GERMANY.2.5.4.6

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Modified Randomized Set

Dyspnoea

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	0.00 (0.000)	-16.67 (23.570)	0.00 (NA)	0.00 (NA)	0.00 (0.000)	-11.11 (19.245)
Median	0.00	-16.67	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	-33.3, 0.0

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Modified Randomized Set

Insomnia

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		15		50	
Mean (SD)	22.86 (22.537)		24.44 (23.458)		23.33 (22.588)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 66.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	33	33	15	15		
Mean (SD)	17.17 (23.748)	-6.06 (21.171)	22.22 (27.217)	-2.22 (26.627)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

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Modified Randomized Set

Insomnia

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	35	35	15	15		
Mean (SD)	17.14 (26.036)	-5.71 (20.589)	24.44 (29.457)	0.00 (30.861)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 66.7		
Week 8						
n	29	29	12	12		
Mean (SD)	12.64 (20.728)	-10.34 (20.125)	13.89 (17.164)	-5.56 (23.925)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 33.3	-33.3, 33.3		

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Modified Randomized Set

Insomnia

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	32	32	15	15		
Mean (SD)	13.54 (18.664)	-10.42 (19.743)	31.11 (34.427)	6.67 (38.214)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-66.7, 66.7		
Week 24						
n	29	29	14	14		
Mean (SD)	13.79 (20.925)	-8.05 (26.209)	16.67 (17.296)	-7.14 (29.753)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 66.7	-66.7, 66.7	0.0, 33.3	-66.7, 33.3		

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Modified Randomized Set

Insomnia

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	12	12	41	41
Mean (SD)	18.39 (21.056)	-4.60 (23.101)	25.00 (32.177)	0.00 (34.816)	20.33 (24.583)	-3.25 (26.672)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7
Week 56						
n	21	21	10	10	31	31
Mean (SD)	15.87 (20.053)	-3.17 (25.614)	33.33 (31.427)	6.67 (30.631)	21.51 (25.164)	0.00 (27.217)
Median	0.00	0.00	33.33	0.00	33.33	0.00
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 66.7

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Modified Randomized Set

Insomnia

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	7	7	19	19
Mean (SD)	8.33 (15.076)	-8.33 (15.076)	28.57 (29.991)	4.76 (40.500)	15.79 (23.223)	-3.51 (26.977)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-66.7, 66.7	0.0, 66.7	-66.7, 66.7
Week 88						
n	8	8	5	5	13	13
Mean (SD)	4.17 (11.785)	-8.33 (15.430)	33.33 (47.140)	6.67 (49.441)	15.38 (32.247)	-2.56 (31.802)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7

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Modified Randomized Set

Insomnia

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	16.67 (23.570)	0.00 (0.000)	100.00 (NA)	66.67 (NA)	44.44 (50.918)	22.22 (38.490)
Median	16.67	0.00	100.00	66.67	33.33	0.00
Min, Max	0.0, 33.3	0.0, 0.0	100.0, 100.0	66.7, 66.7	0.0, 100.0	0.0, 66.7

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Appetite Loss

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		15		50	
Mean (SD)	10.48 (17.660)		17.78 (30.516)		12.67 (22.223)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 100.0		0.0, 100.0	
Week 2						
n	34	34	15	15		
Mean (SD)	13.73 (21.893)	2.94 (20.707)	20.00 (24.560)	2.22 (19.787)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-33.3, 33.3		

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Appetite Loss

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	35	35	15	15		
Mean (SD)	8.57 (16.848)	-1.90 (16.053)	15.56 (30.516)	-2.22 (26.627)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-66.7, 66.7		
Week 8						
n	29	29	12	12		
Mean (SD)	16.09 (19.150)	3.45 (20.596)	8.33 (15.076)	-8.33 (32.177)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 33.3	-100.0, 33.3		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:28

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Appetite Loss

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	32	32	15	15		
Mean (SD)	9.38 (21.140)	-1.04 (27.415)	15.56 (24.774)	-2.22 (26.627)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 66.7	-66.7, 33.3		
Week 24						
n	29	29	14	14		
Mean (SD)	11.49 (22.318)	1.15 (30.188)	11.90 (21.111)	0.00 (13.074)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 100.0	0.0, 66.7	-33.3, 33.3		

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Appetite Loss

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	12	12	41	41
Mean (SD)	8.05 (14.516)	0.00 (15.430)	22.22 (32.824)	13.89 (30.011)	12.20 (22.056)	4.07 (21.337)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	0.0, 100.0	0.0, 100.0	-33.3, 100.0
Week 56						
n	21	21	10	10	31	31
Mean (SD)	6.35 (13.412)	0.00 (18.257)	10.00 (16.102)	0.00 (0.000)	7.53 (14.167)	0.00 (14.907)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	0.0, 0.0	0.0, 33.3	-33.3, 33.3

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Appetite Loss

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	7	7	19	19
Mean (SD)	2.78 (9.623)	-5.56 (12.975)	19.05 (26.227)	9.52 (25.198)	8.77 (18.732)	0.00 (19.245)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 66.7	0.0, 66.7	0.0, 66.7	-33.3, 66.7
Week 88						
n	8	8	5	5	13	13
Mean (SD)	4.17 (11.785)	-4.17 (11.785)	13.33 (18.257)	6.67 (14.907)	7.69 (14.618)	0.00 (13.608)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	0.0, 33.3	0.0, 33.3	-33.3, 33.3

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Appetite Loss

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	0.00 (0.000)	0.00 (0.000)	66.67 (NA)	33.33 (NA)	22.22 (38.490)	11.11 (19.245)
Median	0.00	0.00	66.67	33.33	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	33.3, 33.3	0.0, 66.7	0.0, 33.3

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Constipation

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		15		50	
Mean (SD)	16.19 (24.749)		8.89 (15.258)		14.00 (22.426)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 33.3		0.0, 100.0	
Week 2						
n	33	33	15	15		
Mean (SD)	7.07 (19.996)	-10.10 (21.221)	15.56 (27.794)	6.67 (25.820)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Constipation

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	35	35	15	15		
Mean (SD)	7.62 (19.944)	-8.57 (23.351)	22.22 (27.217)	13.33 (21.082)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	0.0, 66.7		
Week 8						
n	29	29	12	12		
Mean (SD)	6.90 (16.377)	-8.05 (21.185)	13.89 (26.432)	8.33 (15.076)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	0.0, 33.3		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Constipation

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	32	32	15	15		
Mean (SD)	8.33 (22.401)	-9.38 (19.371)	17.78 (30.516)	8.89 (19.787)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	0.0, 66.7		
Week 24						
n	29	29	14	14		
Mean (SD)	6.90 (20.662)	-10.34 (22.009)	16.67 (28.495)	7.14 (23.310)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties. Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem. Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Constipation

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	12	12	41	41
Mean (SD)	4.60 (11.698)	-13.79 (22.743)	13.89 (22.285)	5.56 (12.975)	7.32 (15.833)	-8.13 (22.086)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	0.0, 33.3	0.0, 66.7	-66.7, 33.3
Week 56						
n	21	21	10	10	31	31
Mean (SD)	9.52 (23.905)	-11.11 (21.943)	6.67 (14.055)	-3.33 (10.541)	8.60 (21.025)	-8.60 (19.183)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 0.0	0.0, 33.3	-33.3, 0.0	0.0, 100.0	-66.7, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Constipation

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	7	7	19	19
Mean (SD)	11.11 (29.588)	-11.11 (16.412)	14.29 (17.817)	4.76 (23.002)	12.28 (25.363)	-5.26 (20.071)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-33.3, 0.0	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 33.3
Week 88						
n	8	8	5	5	13	13
Mean (SD)	12.50 (17.252)	0.00 (17.817)	40.00 (54.772)	33.33 (47.140)	23.08 (36.980)	12.82 (34.797)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	0.0, 100.0	0.0, 100.0	-33.3, 100.0

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Constipation

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	16.67 (23.570)	0.00 (0.000)	66.67 (NA)	33.33 (NA)	33.33 (33.333)	11.11 (19.245)
Median	16.67	0.00	66.67	33.33	33.33	0.00
Min, Max	0.0, 33.3	0.0, 0.0	66.7, 66.7	33.3, 33.3	0.0, 66.7	0.0, 33.3

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Modified Randomized Set

Diarrhea

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		15		50	
Mean (SD)	3.81 (10.760)		11.11 (20.574)		6.00 (14.584)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 33.3		0.0, 66.7		0.0, 66.7	
Week 2						
n	34	34	15	15		
Mean (SD)	3.92 (13.645)	0.00 (8.206)	4.44 (17.213)	-6.67 (22.537)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-66.7, 33.3		

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Modified Randomized Set

Diarrhea

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	35	35	15	15		
Mean (SD)	4.76 (11.835)	0.95 (15.094)	2.22 (8.607)	-8.89 (15.258)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 0.0		
Week 8						
n	29	29	12	12		
Mean (SD)	8.05 (17.032)	4.60 (19.363)	5.56 (19.245)	-8.33 (15.076)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-33.3, 0.0		

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Modified Randomized Set

Diarrhea

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	32	32	15	15		
Mean (SD)	5.21 (12.297)	1.04 (15.804)	4.44 (17.213)	-6.67 (28.730)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-66.7, 66.7		
Week 24						
n	29	29	14	14		
Mean (SD)	4.60 (11.698)	2.30 (12.380)	4.76 (12.105)	-4.76 (17.817)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-66.7, 0.0		

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Modified Randomized Set

Diarrhea

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	12	12	41	41
Mean (SD)	3.45 (10.331)	-1.15 (16.626)	5.56 (12.975)	-5.56 (19.245)	4.07 (11.043)	-2.44 (17.305)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3
Week 56						
n	21	21	10	10	31	31
Mean (SD)	6.35 (13.412)	4.76 (11.952)	3.33 (10.541)	-10.00 (27.442)	5.38 (12.463)	0.00 (19.245)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Diarrhea

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	7	7	19	19
Mean (SD)	0.00 (0.000)	-2.78 (9.623)	14.29 (17.817)	-4.76 (23.002)	5.26 (12.488)	-3.51 (15.294)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3
Week 88						
n	8	8	5	5	13	13
Mean (SD)	0.00 (0.000)	0.00 (0.000)	6.67 (14.907)	-13.33 (38.006)	2.56 (9.245)	-5.13 (22.958)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3

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Modified Randomized Set

Diarrhea

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (NA)	0.00 (NA)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0

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Modified Randomized Set

Financial Difficulties

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		15		50	
Mean (SD)	21.90 (31.252)		17.78 (30.516)		20.67 (30.780)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	35	35	15	15		
Mean (SD)	18.10 (27.228)	-3.81 (15.700)	15.56 (27.794)	-2.22 (19.787)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 33.3		

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Modified Randomized Set

Financial Difficulties

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	35	35	15	15		
Mean (SD)	17.14 (24.749)	-4.76 (18.334)	8.89 (26.627)	-8.89 (23.458)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 33.3		
Week 8						
n	29	29	12	12		
Mean (SD)	11.49 (18.422)	-6.90 (25.788)	11.11 (21.711)	2.78 (17.164)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-100.0, 33.3	0.0, 66.7	-33.3, 33.3		

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Financial Difficulties

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	32	32	15	15		
Mean (SD)	18.75 (25.312)	-4.17 (20.302)	17.78 (33.014)	0.00 (28.172)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 66.7		
Week 24						
n	29	29	14	14		
Mean (SD)	14.94 (24.537)	-8.05 (24.649)	11.90 (30.959)	-7.14 (23.310)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 100.0	-66.7, 33.3		

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Modified Randomized Set

Financial Difficulties

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	12	12	41	41
Mean (SD)	18.39 (26.105)	-6.90 (24.200)	5.56 (12.975)	-16.67 (30.151)	14.63 (23.628)	-9.76 (26.081)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-100.0, 0.0	0.0, 100.0	-100.0, 33.3
Week 56						
n	21	21	10	10	31	31
Mean (SD)	17.46 (27.119)	-9.52 (18.687)	20.00 (35.832)	-6.67 (26.294)	18.28 (29.613)	-8.60 (21.025)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 33.3

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Modified Randomized Set

Financial Difficulties

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	7	7	19	19
Mean (SD)	11.11 (16.412)	-8.33 (25.126)	4.76 (12.599)	-14.29 (26.227)	8.77 (15.080)	-10.53 (24.976)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 33.3
Week 88						
n	8	8	5	5	13	13
Mean (SD)	12.50 (17.252)	-4.17 (11.785)	0.00 (0.000)	-20.00 (29.814)	7.69 (14.618)	-10.26 (21.014)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 0.0	-66.7, 0.0	0.0, 33.3	-66.7, 0.0

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Modified Randomized Set

Financial Difficulties

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	0.00 (0.000)	-33.33 (47.140)	0.00 (NA)	-66.67 (NA)	0.00 (0.000)	-44.44 (38.490)
Median	0.00	-33.33	0.00	-66.67	0.00	-66.67
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 0.0	-66.7, -66.7	0.0, 0.0	-66.7, 0.0

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Modified Randomized Set

Fatigue

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	38.38 (32.299)		49.38 (27.839)		41.58 (31.027)	
Median	33.33		44.44		33.33	
Min, Max	0.0, 100.0		11.1, 100.0		0.0, 100.0	
Week 2						
n	22	22	10	8		
Mean (SD)	31.31 (31.359)	-7.07 (20.443)	47.78 (29.653)	5.56 (24.488)		
Median	22.22	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-44.4, 33.3	11.1, 100.0	-33.3, 44.4		

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Modified Randomized Set

Fatigue

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	9		
Mean (SD)	25.76 (28.971)	-12.63 (26.626)	41.11 (20.320)	-4.94 (22.981)		
Median	16.67	-11.11	33.33	0.00		
Min, Max	0.0, 100.0	-55.6, 44.4	11.1, 77.8	-44.4, 22.2		
Week 8						
n	16	16	7	6		
Mean (SD)	15.28 (13.380)	-23.61 (27.179)	38.10 (14.138)	-5.56 (11.653)		
Median	11.11	-22.22	33.33	-5.56		
Min, Max	0.0, 33.3	-66.7, 22.2	22.2, 55.6	-22.2, 11.1		

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Modified Randomized Set

Fatigue

Screening Hgb Level \geq 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	9		
Mean (SD)	22.78 (22.068)	-16.11 (22.068)	51.11 (28.304)	-1.23 (31.644)		
Median	22.22	-11.11	38.89	-11.11		
Min, Max	0.0, 66.7	-66.7, 22.2	22.2, 100.0	-33.3, 77.8		
Week 24						
n	20	20	10	9		
Mean (SD)	26.11 (27.519)	-12.78 (27.754)	40.00 (25.229)	-8.64 (36.758)		
Median	22.22	0.00	33.33	-11.11		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 88.9	-44.4, 77.8		

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Modified Randomized Set

Fatigue

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	9	9	26	26
Mean (SD)	25.49 (27.150)	-14.38 (22.827)	44.44 (27.778)	-4.94 (26.708)	32.05 (28.341)	-11.11 (24.140)
Median	22.22	-11.11	33.33	0.00	33.33	-11.11
Min, Max	0.0, 100.0	-55.6, 33.3	11.1, 100.0	-55.6, 33.3	0.0, 100.0	-55.6, 33.3
Week 56						
n	13	13	7	6	20	19
Mean (SD)	30.77 (28.384)	-10.26 (31.250)	38.10 (14.138)	-12.96 (35.428)	33.33 (24.183)	-11.11 (31.644)
Median	22.22	0.00	33.33	-11.11	33.33	-11.11
Min, Max	0.0, 100.0	-66.7, 44.4	22.2, 55.6	-66.7, 44.4	0.0, 100.0	-66.7, 44.4

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Modified Randomized Set

Fatigue

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	5	4	14	13
Mean (SD)	25.93 (24.216)	-16.05 (18.519)	51.11 (16.851)	-11.11 (27.217)	34.92 (24.598)	-14.53 (20.480)
Median	22.22	-22.22	55.56	-16.67	33.33	-22.22
Min, Max	0.0, 66.7	-33.3, 22.2	33.3, 66.7	-33.3, 22.2	0.0, 66.7	-33.3, 22.2
Week 88						
n	6	6	3	3	9	9
Mean (SD)	27.78 (28.760)	-12.96 (29.327)	40.74 (12.830)	-37.04 (27.962)	32.10 (24.498)	-20.99 (29.630)
Median	22.22	-16.67	33.33	-33.33	33.33	-33.33
Min, Max	0.0, 66.7	-44.4, 33.3	33.3, 55.6	-66.7, -11.1	0.0, 66.7	-66.7, 33.3

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Fatigue

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	16.67 (23.570)	-27.78 (39.284)	22.22 (NA)	-77.78 (NA)	18.52 (16.973)	-44.44 (40.062)
Median	16.67	-27.78	22.22	-77.78	22.22	-55.56
Min, Max	0.0, 33.3	-55.6, 0.0	22.2, 22.2	-77.8, -77.8	0.0, 33.3	-77.8, 0.0

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Modified Randomized Set

Nausea and Vomiting

Screening Hgb Level \geq 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	3.79 (14.486)		5.56 (11.785)		4.30 (13.586)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 33.3		0.0, 66.7	
Week 2						
n	22	22	10	8		
Mean (SD)	4.55 (11.708)	0.76 (15.834)	8.33 (14.164)	0.00 (8.909)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 50.0	-50.0, 50.0	0.0, 33.3	-16.7, 16.7		

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Modified Randomized Set

Nausea and Vomiting

Screening Hgb Level \geq 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	9		
Mean (SD)	4.55 (9.175)	0.76 (14.065)	3.33 (10.541)	-1.85 (13.029)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-50.0, 33.3	0.0, 33.3	-33.3, 16.7		
Week 8						
n	16	16	7	6		
Mean (SD)	4.17 (9.623)	-1.04 (9.562)	9.52 (18.898)	8.33 (13.944)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 16.7	0.0, 50.0	0.0, 33.3		

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Modified Randomized Set

Nausea and Vomiting

Screening Hgb Level \geq 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	9		
Mean (SD)	3.33 (8.719)	0.00 (14.307)	16.67 (28.328)	7.41 (16.897)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-50.0, 33.3	0.0, 83.3	0.0, 50.0		
Week 24						
n	20	20	10	9		
Mean (SD)	5.00 (15.390)	1.67 (19.421)	5.00 (8.051)	0.00 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-50.0, 66.7	0.0, 16.7	-33.3, 16.7		

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Modified Randomized Set

Nausea and Vomiting

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	9	9	26	26
Mean (SD)	4.90 (7.828)	3.92 (7.287)	3.70 (7.349)	-1.85 (10.015)	4.49 (7.539)	1.92 (8.598)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	0.0, 16.7	0.0, 16.7	-16.7, 16.7	0.0, 16.7	-16.7, 16.7
Week 56						
n	13	13	7	6	20	19
Mean (SD)	1.28 (4.623)	0.00 (6.804)	4.76 (8.133)	-2.78 (16.387)	2.50 (6.106)	-0.88 (10.354)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	-16.7, 16.7	0.0, 16.7	-33.3, 16.7	0.0, 16.7	-33.3, 16.7

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Modified Randomized Set

Nausea and Vomiting

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	5	4	14	13
Mean (SD)	1.85 (5.556)	0.00 (8.333)	6.67 (9.129)	-4.17 (25.000)	3.57 (7.097)	-1.28 (14.372)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	-16.7, 16.7	0.0, 16.7	-33.3, 16.7	0.0, 16.7	-33.3, 16.7
Week 88						
n	6	6	3	3	9	9
Mean (SD)	2.78 (6.804)	0.00 (0.000)	11.11 (9.623)	-5.56 (25.459)	5.56 (8.333)	-1.85 (13.029)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 16.7	0.0, 0.0	0.0, 16.7	-33.3, 16.7	0.0, 16.7	-33.3, 16.7

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Modified Randomized Set

Nausea and Vomiting

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	0.00 (0.000)	0.00 (0.000)	66.67 (NA)	33.33 (NA)	22.22 (38.490)	11.11 (19.245)
Median	0.00	0.00	66.67	33.33	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	33.3, 33.3	0.0, 66.7	0.0, 33.3

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Modified Randomized Set

Pain

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	17.42 (26.466)		12.96 (18.215)		16.13 (24.146)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 50.0		0.0, 100.0	
Week 2						
n	22	22	10	8		
Mean (SD)	12.88 (25.162)	-4.55 (13.784)	25.00 (19.642)	8.33 (21.822)		
Median	0.00	0.00	16.67	8.33		
Min, Max	0.0, 100.0	-33.3, 16.7	0.0, 50.0	-33.3, 33.3		

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Modified Randomized Set

Pain

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	9		
Mean (SD)	17.42 (25.962)	0.00 (13.608)	21.67 (31.476)	11.11 (28.868)		
Median	0.00	0.00	8.33	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-16.7, 83.3		
Week 8						
n	16	16	7	6		
Mean (SD)	8.33 (14.907)	-6.25 (18.130)	11.90 (12.599)	-2.78 (16.387)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 50.0	-33.3, 33.3	0.0, 33.3	-33.3, 16.7		

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Pain

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	9		
Mean (SD)	15.83 (29.357)	-0.83 (15.742)	33.33 (34.247)	20.37 (37.060)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 83.3		
Week 24						
n	20	20	10	9		
Mean (SD)	20.00 (33.595)	3.33 (19.941)	15.00 (18.342)	3.70 (13.889)		
Median	0.00	0.00	8.33	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 50.0	-16.7, 33.3		

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Pain

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	9	9	26	26
Mean (SD)	19.61 (27.786)	0.00 (15.590)	27.78 (31.180)	14.81 (36.747)	22.44 (28.652)	5.13 (25.285)
Median	0.00	0.00	33.33	0.00	8.33	0.00
Min, Max	0.0, 83.3	-33.3, 33.3	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0
Week 56						
n	13	13	7	6	20	19
Mean (SD)	25.64 (34.437)	1.28 (14.372)	23.81 (23.288)	11.11 (29.187)	25.00 (30.349)	4.39 (19.909)
Median	0.00	0.00	16.67	0.00	16.67	0.00
Min, Max	0.0, 100.0	-16.7, 33.3	0.0, 66.7	-16.7, 66.7	0.0, 100.0	-16.7, 66.7

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Pain

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	5	4	13	12
Mean (SD)	10.42 (23.465)	-8.33 (15.430)	16.67 (16.667)	0.00 (23.570)	12.82 (20.586)	-5.56 (17.885)
Median	0.00	0.00	16.67	-8.33	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 33.3	-16.7, 33.3	0.0, 66.7	-33.3, 33.3
Week 88						
n	6	6	3	3	9	9
Mean (SD)	16.67 (27.889)	-8.33 (13.944)	22.22 (25.459)	16.67 (28.868)	18.52 (25.610)	0.00 (22.048)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 50.0	0.0, 50.0	0.0, 66.7	-33.3, 50.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Pain

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	16.67 (23.570)	-33.33 (47.140)	16.67 (NA)	0.00 (NA)	16.67 (16.667)	-22.22 (38.490)
Median	16.67	-33.33	16.67	0.00	16.67	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	16.7, 16.7	0.0, 0.0	0.0, 33.3	-66.7, 0.0

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Modified Randomized Set

Dyspnoea

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	33.33 (27.217)		25.93 (27.778)		31.18 (27.129)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	22	22	10	8		
Mean (SD)	18.18 (22.366)	-15.15 (19.860)	40.00 (34.427)	20.83 (24.801)		
Median	0.00	0.00	33.33	16.67		
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 100.0	0.0, 66.7		

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Modified Randomized Set

Dyspnoea

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	9		
Mean (SD)	7.58 (14.298)	-25.76 (22.845)	33.33 (41.574)	11.11 (23.570)		
Median	0.00	-33.33	16.67	0.00		
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 100.0	0.0, 66.7		
Week 8						
n	16	16	7	6		
Mean (SD)	6.25 (13.437)	-29.17 (23.960)	23.81 (25.198)	-5.56 (13.608)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 33.3	-100.0, 0.0	0.0, 66.7	-33.3, 0.0		

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Modified Randomized Set

Dyspnoea

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	9		
Mean (SD)	11.67 (22.361)	-21.67 (27.091)	26.67 (26.294)	0.00 (33.333)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-33.3, 66.7		
Week 24						
n	20	20	10	9		
Mean (SD)	16.67 (25.363)	-16.67 (31.530)	16.67 (17.568)	-7.41 (27.778)		
Median	0.00	-33.33	16.67	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 33.3	-66.7, 33.3		

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Modified Randomized Set

Dyspnoea

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	9	9	26	26
Mean (SD)	17.65 (23.914)	-17.65 (17.150)	18.52 (17.568)	-7.41 (22.222)	17.95 (21.563)	-14.10 (19.260)
Median	0.00	-33.33	33.33	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-33.3, 33.3
Week 56						
n	13	13	7	6	20	19
Mean (SD)	30.77 (31.802)	-2.56 (25.318)	9.52 (16.265)	0.00 (21.082)	23.33 (28.817)	-1.75 (23.501)
Median	33.33	0.00	0.00	0.00	16.67	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 33.3

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Dyspnoea

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	5	4	14	13
Mean (SD)	14.81 (24.216)	-18.52 (17.568)	13.33 (18.257)	-8.33 (16.667)	14.29 (21.540)	-15.38 (17.296)
Median	0.00	-33.33	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-33.3, 0.0
Week 88						
n	6	6	3	3	9	9
Mean (SD)	11.11 (27.217)	-16.67 (27.889)	0.00 (0.000)	-11.11 (19.245)	7.41 (22.222)	-14.81 (24.216)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 0.0	-33.3, 0.0	0.0, 66.7	-66.7, 0.0

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Modified Randomized Set

Dyspnoea

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	0.00 (0.000)	-33.33 (47.140)	33.33 (NA)	0.00 (NA)	11.11 (19.245)	-22.22 (38.490)
Median	0.00	-33.33	33.33	0.00	0.00	0.00
Min, Max	0.0, 0.0	-66.7, 0.0	33.3, 33.3	0.0, 0.0	0.0, 33.3	-66.7, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Insomnia

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	31.82 (34.850)		37.04 (42.310)		33.33 (36.515)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	22	22	10	8		
Mean (SD)	28.79 (37.509)	-3.03 (17.545)	40.00 (37.843)	4.17 (41.547)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 66.7		

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Modified Randomized Set

Insomnia

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	9		
Mean (SD)	27.27 (37.987)	-4.55 (29.628)	26.67 (34.427)	-11.11 (44.096)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-100.0, 33.3		
Week 8						
n	16	16	7	6		
Mean (SD)	14.58 (17.078)	-16.67 (32.203)	19.05 (37.796)	-11.11 (45.542)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 100.0	-100.0, 33.3		

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Modified Randomized Set

Insomnia

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	9		
Mean (SD)	21.67 (31.110)	-8.33 (21.289)	30.00 (33.148)	-7.41 (27.778)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 0.0	0.0, 100.0	-66.7, 33.3		
Week 24						
n	20	20	10	9		
Mean (SD)	16.67 (31.530)	-13.33 (31.344)	26.67 (30.631)	-11.11 (16.667)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 66.7	-33.3, 0.0		

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Modified Randomized Set

Insomnia

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	9	9	26	26
Mean (SD)	23.53 (36.827)	-9.80 (25.725)	25.93 (36.430)	-11.11 (47.140)	24.36 (35.972)	-10.26 (33.690)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-100.0, 66.7	0.0, 100.0	-100.0, 66.7
Week 56						
n	13	13	7	6	20	19
Mean (SD)	30.77 (34.592)	-5.13 (26.688)	42.86 (37.090)	-5.56 (38.968)	35.00 (35.002)	-5.26 (29.945)
Median	33.33	0.00	33.33	0.00	33.33	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 33.3

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Modified Randomized Set

Insomnia

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	5	4	14	13
Mean (SD)	18.52 (24.216)	-18.52 (24.216)	53.33 (38.006)	-16.67 (63.828)	30.95 (33.242)	-17.95 (37.553)
Median	0.00	0.00	66.67	0.00	33.33	0.00
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 100.0	-100.0, 33.3	0.0, 100.0	-100.0, 33.3
Week 88						
n	6	6	3	3	9	9
Mean (SD)	16.67 (27.889)	-16.67 (18.257)	55.56 (38.490)	-11.11 (50.918)	29.63 (35.136)	-14.81 (29.397)
Median	0.00	-16.67	33.33	0.00	33.33	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	33.3, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 33.3

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Modified Randomized Set

Insomnia

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	0.00 (0.000)	-33.33 (47.140)	0.00 (NA)	-100.00 (NA)	0.00 (0.000)	-55.56 (50.918)
Median	0.00	-33.33	0.00	-100.00	0.00	-66.67
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 0.0	-100.0, -100.0	0.0, 0.0	-100.0, 0.0

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Modified Randomized Set

Appetite Loss

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	6.06 (13.159)		18.52 (24.216)		9.68 (17.625)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 33.3		0.0, 66.7		0.0, 66.7	
Week 2						
n	22	22	10	8		
Mean (SD)	4.55 (11.708)	-1.52 (16.191)	20.00 (23.307)	0.00 (0.000)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	0.0, 0.0		

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Appetite Loss

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	9		
Mean (SD)	1.52 (7.107)	-4.55 (11.708)	10.00 (22.498)	-7.41 (27.778)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-66.7, 33.3		
Week 8						
n	16	16	7	6		
Mean (SD)	8.33 (19.245)	0.00 (21.082)	19.05 (26.227)	5.56 (13.608)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	0.0, 33.3		

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Modified Randomized Set

Appetite Loss

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	9		
Mean (SD)	3.33 (14.907)	-1.67 (20.160)	20.00 (35.832)	3.70 (20.031)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 100.0	-33.3, 33.3		
Week 24						
n	20	20	10	9		
Mean (SD)	10.00 (24.423)	5.00 (27.091)	23.33 (27.442)	7.41 (14.699)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 66.7	0.0, 33.3		

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Appetite Loss

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	9	9	26	26
Mean (SD)	3.92 (11.070)	-1.96 (18.524)	14.81 (17.568)	-3.70 (20.031)	7.69 (14.322)	-2.56 (18.674)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3
Week 56						
n	13	13	7	6	20	19
Mean (SD)	10.26 (16.013)	5.13 (22.958)	14.29 (37.796)	-5.56 (44.305)	11.67 (24.839)	1.75 (30.376)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Table GERMANY.2.5.4.6

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Modified Randomized Set

Appetite Loss

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	5	4	14	13
Mean (SD)	3.70 (11.111)	-3.70 (11.111)	20.00 (18.257)	-8.33 (16.667)	9.52 (15.627)	-5.13 (12.518)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 0.0
Week 88						
n	6	6	3	3	9	9
Mean (SD)	11.11 (17.213)	5.56 (13.608)	22.22 (19.245)	-11.11 (50.918)	14.81 (17.568)	0.00 (28.868)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3

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Appetite Loss

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	0.00 (0.000)	0.00 (0.000)	66.67 (NA)	0.00 (NA)	22.22 (38.490)	0.00 (0.000)
Median	0.00	0.00	66.67	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	0.0, 0.0	0.0, 66.7	0.0, 0.0

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Modified Randomized Set

Constipation

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	9.09 (21.036)		14.81 (24.216)		10.75 (21.751)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	22	22	10	8		
Mean (SD)	13.64 (26.546)	4.55 (11.708)	20.00 (35.832)	4.17 (27.817)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	0.0, 33.3	0.0, 100.0	-33.3, 66.7		

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Modified Randomized Set

Constipation

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	9		
Mean (SD)	13.64 (26.546)	4.55 (15.585)	13.33 (32.203)	0.00 (28.868)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	0.0, 66.7	0.0, 100.0	-33.3, 66.7		
Week 8						
n	16	16	7	6		
Mean (SD)	4.17 (11.386)	-4.17 (20.638)	19.05 (37.796)	5.56 (13.608)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 100.0	0.0, 33.3		

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Modified Randomized Set

Constipation

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	9		
Mean (SD)	11.67 (27.091)	5.00 (19.571)	16.67 (36.004)	3.70 (26.058)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 66.7		
Week 24						
n	20	20	10	9		
Mean (SD)	6.67 (17.438)	0.00 (15.294)	16.67 (23.570)	3.70 (20.031)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3		

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Modified Randomized Set

Constipation

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	9	9	26	26
Mean (SD)	9.80 (19.596)	0.00 (16.667)	18.52 (24.216)	3.70 (30.932)	12.82 (21.243)	1.28 (22.072)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-33.3, 66.7
Week 56						
n	13	13	7	6	20	19
Mean (SD)	10.26 (21.014)	-2.56 (16.452)	19.05 (26.227)	5.56 (13.608)	13.33 (22.685)	0.00 (15.713)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 66.7	0.0, 33.3	0.0, 66.7	-33.3, 33.3

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Modified Randomized Set

Constipation

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	5	4	14	13
Mean (SD)	14.81 (17.568)	-3.70 (26.058)	6.67 (14.907)	0.00 (0.000)	11.90 (16.575)	-2.56 (21.350)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	0.0, 0.0	0.0, 33.3	-33.3, 33.3
Week 88						
n	6	6	3	3	9	9
Mean (SD)	16.67 (18.257)	-11.11 (27.217)	0.00 (0.000)	-11.11 (19.245)	11.11 (16.667)	-11.11 (23.570)
Median	16.67	-16.67	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 0.0	-33.3, 0.0	0.0, 33.3	-33.3, 33.3

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Modified Randomized Set

Constipation

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	16.67 (23.570)	-16.67 (23.570)	0.00 (NA)	-33.33 (NA)	11.11 (19.245)	-22.22 (19.245)
Median	16.67	-16.67	0.00	-33.33	0.00	-33.33
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 0.0	-33.3, -33.3	0.0, 33.3	-33.3, 0.0

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and \geq 8.5 g/dL)
Modified Randomized Set

Diarrhea

Screening Hgb Level \geq 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	7.58 (22.845)		14.81 (24.216)		9.68 (23.084)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	22	22	10	8		
Mean (SD)	9.09 (23.417)	1.52 (26.181)	6.67 (14.055)	-8.33 (23.570)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 100.0	0.0, 33.3	-33.3, 33.3		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Diarrhea

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	9		
Mean (SD)	6.06 (16.703)	-1.52 (26.181)	6.67 (14.055)	-7.41 (27.778)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-100.0, 66.7	0.0, 33.3	-66.7, 33.3		
Week 8						
n	16	16	7	6		
Mean (SD)	6.25 (13.437)	-2.08 (19.124)	9.52 (16.265)	-5.56 (25.092)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-33.3, 33.3		

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Modified Randomized Set

Diarrhea

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	9		
Mean (SD)	6.67 (13.680)	-1.67 (17.014)	3.33 (10.541)	-11.11 (28.868)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3		
Week 24						
n	20	20	10	9		
Mean (SD)	6.67 (13.680)	-1.67 (20.160)	3.33 (10.541)	-11.11 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-33.3, 0.0		

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Modified Randomized Set

Diarrhea

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	9	9	26	26
Mean (SD)	7.84 (14.575)	-1.96 (27.565)	11.11 (16.667)	-3.70 (30.932)	8.97 (15.078)	-2.56 (28.163)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-100.0, 33.3
Week 56						
n	13	13	7	6	20	19
Mean (SD)	7.69 (14.618)	-2.56 (31.802)	14.29 (17.817)	-11.11 (17.213)	10.00 (15.672)	-5.26 (27.807)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-100.0, 33.3

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Modified Randomized Set

Diarrhea

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	5	4	13	12
Mean (SD)	0.00 (0.000)	0.00 (0.000)	40.00 (43.461)	25.00 (50.000)	15.38 (32.247)	8.33 (28.868)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 100.0	0.0, 100.0	0.0, 100.0	0.0, 100.0
Week 88						
n	6	6	3	3	9	9
Mean (SD)	5.56 (13.608)	5.56 (13.608)	11.11 (19.245)	0.00 (0.000)	7.41 (14.699)	3.70 (11.111)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 0.0	0.0, 33.3	0.0, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Diarrhea

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (NA)	0.00 (NA)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0

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Modified Randomized Set

Financial Difficulties

Screening Hgb Level \geq 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	18.18 (30.389)		33.33 (33.333)		22.58 (31.490)	
Median	0.00		33.33		0.00	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	22	22	10	8		
Mean (SD)	18.18 (33.692)	0.00 (14.548)	36.67 (39.907)	12.50 (39.591)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 100.0		

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Modified Randomized Set

Financial Difficulties

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	9		
Mean (SD)	18.18 (30.389)	0.00 (23.002)	33.33 (38.490)	3.70 (38.889)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-33.3, 100.0		
Week 8						
n	16	16	7	6		
Mean (SD)	14.58 (27.131)	-6.25 (13.437)	19.05 (26.227)	-5.56 (25.092)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 0.0	0.0, 66.7	-33.3, 33.3		

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Modified Randomized Set

Financial Difficulties

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	9		
Mean (SD)	16.67 (29.617)	0.00 (10.815)	30.00 (39.907)	0.00 (40.825)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 100.0		
Week 24						
n	20	20	10	9		
Mean (SD)	21.67 (34.666)	5.00 (24.839)	36.67 (36.683)	7.41 (36.430)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0		

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Modified Randomized Set

Financial Difficulties

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	9	9	26	26
Mean (SD)	15.69 (29.149)	-1.96 (21.955)	40.74 (40.062)	7.41 (40.062)	24.36 (34.715)	1.28 (29.030)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-66.7, 100.0
Week 56						
n	13	13	7	6	20	19
Mean (SD)	5.13 (12.518)	-7.69 (19.971)	33.33 (33.333)	5.56 (57.413)	15.00 (25.305)	-3.51 (34.951)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 100.0	-66.7, 100.0	0.0, 100.0	-66.7, 100.0

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Financial Difficulties

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	5	4	13	12
Mean (SD)	4.17 (11.785)	-12.50 (24.801)	20.00 (29.814)	-16.67 (19.245)	10.26 (21.014)	-13.89 (22.285)
Median	0.00	0.00	0.00	-16.67	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 66.7	-33.3, 0.0	0.0, 66.7	-66.7, 0.0
Week 88						
n	6	6	3	3	9	9
Mean (SD)	5.56 (13.608)	-16.67 (27.889)	22.22 (19.245)	-33.33 (33.333)	11.11 (16.667)	-22.22 (28.868)
Median	0.00	0.00	33.33	-33.33	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0

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Table GERMANY.2.5.4.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Financial Difficulties

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	0.00 (0.000)	0.00 (0.000)	33.33 (NA)	-66.67 (NA)	11.11 (19.245)	-22.22 (38.490)
Median	0.00	0.00	33.33	-66.67	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	33.3, 33.3	-66.7, -66.7	0.0, 33.3	-66.7, 0.0

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Table GERMANY.2.5.4.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Fatigue

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		10		31	
Mean (SD)	38.62 (23.470)		42.22 (19.458)		39.78 (21.994)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 77.8		11.1, 77.8		0.0, 77.8	
Week 2						
n	21	21	11	9		
Mean (SD)	34.92 (25.649)	-3.70 (21.469)	43.43 (16.817)	7.41 (20.031)		
Median	33.33	0.00	44.44	0.00		
Min, Max	0.0, 88.9	-55.6, 33.3	11.1, 66.7	-22.2, 44.4		

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Fatigue

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	21	21	11	10		
Mean (SD)	29.63 (23.917)	-8.99 (23.990)	49.49 (25.993)	11.11 (17.372)		
Median	33.33	0.00	33.33	5.56		
Min, Max	0.0, 77.8	-55.6, 44.4	11.1, 100.0	-11.1, 44.4		
Week 8						
n	14	14	6	5		
Mean (SD)	26.19 (19.298)	-11.90 (20.656)	44.44 (19.876)	11.11 (20.787)		
Median	27.78	-11.11	38.89	11.11		
Min, Max	0.0, 66.7	-44.4, 22.2	22.2, 77.8	-11.1, 44.4		

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Fatigue

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	17	17	11	10		
Mean (SD)	29.41 (21.133)	-8.50 (19.055)	58.59 (30.263)	14.44 (33.148)		
Median	33.33	0.00	55.56	5.56		
Min, Max	0.0, 66.7	-44.4, 22.2	11.1, 100.0	-22.2, 77.8		
Week 24						
n	14	14	11	10		
Mean (SD)	30.95 (29.293)	-4.76 (22.943)	39.39 (31.958)	-2.22 (40.164)		
Median	22.22	-5.56	33.33	-5.56		
Min, Max	0.0, 88.9	-33.3, 33.3	0.0, 100.0	-55.6, 77.8		

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Fatigue

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	10	10	27	27
Mean (SD)	30.72 (24.066)	-11.11 (15.713)	45.56 (39.701)	3.33 (38.150)	36.21 (30.908)	-5.76 (26.576)
Median	33.33	-11.11	33.33	0.00	33.33	-11.11
Min, Max	0.0, 77.8	-33.3, 22.2	0.0, 100.0	-44.4, 66.7	0.0, 100.0	-44.4, 66.7
Week 56						
n	12	12	10	9	22	21
Mean (SD)	34.26 (22.453)	-5.56 (23.925)	31.11 (12.614)	-12.35 (26.320)	32.83 (18.298)	-8.47 (24.572)
Median	33.33	-11.11	33.33	-11.11	33.33	-11.11
Min, Max	0.0, 66.7	-33.3, 44.4	11.1, 55.6	-55.6, 44.4	0.0, 66.7	-55.6, 44.4

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Fatigue

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	7	7	8	7	15	14
Mean (SD)	20.63 (21.687)	-17.46 (15.525)	43.06 (24.801)	4.76 (32.620)	32.59 (25.360)	-6.35 (27.117)
Median	22.22	-11.11	33.33	0.00	33.33	-5.56
Min, Max	0.0, 55.6	-33.3, 0.0	22.2, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 66.7
Week 88						
n	5	5	5	5	10	10
Mean (SD)	28.89 (25.580)	-4.44 (25.580)	40.00 (28.974)	-2.22 (32.773)	34.44 (26.424)	-3.33 (27.741)
Median	33.33	0.00	33.33	0.00	33.33	0.00
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 77.8	-33.3, 44.4	0.0, 77.8	-33.3, 44.4

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Table GERMANY.2.5.4.7

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Fatigue

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	-33.33 (NA)	33.33 (NA)	0.00 (NA)	16.67 (23.570)	-16.67 (23.570)
Median	0.00	-33.33	33.33	0.00	16.67	-16.67
Min, Max	0.0, 0.0	-33.3, -33.3	33.3, 33.3	0.0, 0.0	0.0, 33.3	-33.3, 0.0

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Nausea and Vomiting

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		10		31	
Mean (SD)	0.79 (3.637)		1.67 (5.270)		1.08 (4.162)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 16.7		0.0, 16.7		0.0, 16.7	
Week 2						
n	21	21	11	9		
Mean (SD)	3.17 (6.706)	2.38 (5.976)	6.06 (13.484)	1.85 (5.556)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 16.7	0.0, 16.7	0.0, 33.3	0.0, 16.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Nausea and Vomiting

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	21	21	11	10		
Mean (SD)	3.97 (8.983)	3.17 (8.529)	1.52 (5.025)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 16.7	0.0, 0.0		
Week 8						
n	14	14	6	5		
Mean (SD)	7.14 (22.374)	5.95 (22.272)	8.33 (9.129)	10.00 (9.129)		
Median	0.00	0.00	8.33	16.67		
Min, Max	0.0, 83.3	0.0, 83.3	0.0, 16.7	0.0, 16.7		

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Nausea and Vomiting

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	17	17	11	10		
Mean (SD)	0.98 (4.042)	0.98 (4.042)	7.58 (15.570)	1.67 (9.461)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 16.7	0.0, 16.7	0.0, 50.0	-16.7, 16.7		
Week 24						
n	14	14	11	10		
Mean (SD)	3.57 (9.649)	3.57 (9.649)	6.06 (8.409)	5.00 (11.249)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 16.7	-16.7, 16.7		

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Nausea and Vomiting

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	10	10	27	27
Mean (SD)	6.86 (11.871)	5.88 (11.698)	6.67 (16.102)	5.00 (17.656)	6.79 (13.285)	5.56 (13.868)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 50.0	-16.7, 50.0	0.0, 50.0	-16.7, 50.0
Week 56						
n	12	12	10	9	22	21
Mean (SD)	1.39 (4.811)	0.00 (7.107)	13.33 (31.230)	12.96 (27.358)	6.82 (21.614)	5.56 (19.245)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	-16.7, 16.7	0.0, 100.0	0.0, 83.3	0.0, 100.0	-16.7, 83.3

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Nausea and Vomiting

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	7	7	8	7	15	14
Mean (SD)	0.00 (0.000)	-2.38 (6.299)	10.42 (12.400)	11.90 (12.599)	5.56 (10.287)	4.76 (12.105)
Median	0.00	0.00	8.33	16.67	0.00	0.00
Min, Max	0.0, 0.0	-16.7, 0.0	0.0, 33.3	0.0, 33.3	0.0, 33.3	-16.7, 33.3
Week 88						
n	5	5	5	5	10	10
Mean (SD)	3.33 (7.454)	0.00 (0.000)	6.67 (9.129)	6.67 (9.129)	5.00 (8.051)	3.33 (7.027)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	0.0, 0.0	0.0, 16.7	0.0, 16.7	0.0, 16.7	0.0, 16.7

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Nausea and Vomiting

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	33.33 (NA)	33.33 (NA)	16.67 (23.570)	16.67 (23.570)
Median	0.00	0.00	33.33	33.33	16.67	16.67
Min, Max	0.0, 0.0	0.0, 0.0	33.3, 33.3	33.3, 33.3	0.0, 33.3	0.0, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Pain

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		10		31	
Mean (SD)	13.49 (23.345)		28.33 (30.480)		18.28 (26.301)	
Median	0.00		25.00		0.00	
Min, Max	0.0, 83.3		0.0, 100.0		0.0, 100.0	
Week 2						
n	21	21	10	8		
Mean (SD)	11.11 (23.174)	-2.38 (7.968)	30.00 (21.943)	-4.17 (27.817)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 83.3	-33.3, 0.0	0.0, 66.7	-33.3, 33.3		

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Pain

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	21	21	11	10		
Mean (SD)	15.87 (22.655)	2.38 (15.171)	21.21 (25.918)	-5.00 (19.325)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 66.7	-16.7, 50.0	0.0, 83.3	-33.3, 16.7		
Week 8						
n	14	14	5	4		
Mean (SD)	9.52 (26.726)	1.19 (30.984)	10.00 (9.129)	-20.83 (15.957)		
Median	0.00	0.00	16.67	-25.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 16.7	-33.3, 0.0		

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Pain

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	17	17	10	9		
Mean (SD)	8.82 (20.512)	-3.92 (12.541)	25.00 (33.564)	-5.56 (35.355)		
Median	0.00	0.00	8.33	-16.67		
Min, Max	0.0, 66.7	-33.3, 16.7	0.0, 83.3	-33.3, 66.7		
Week 24						
n	14	14	11	10		
Mean (SD)	9.52 (20.375)	-3.57 (14.876)	12.12 (18.395)	-15.00 (27.722)		
Median	0.00	0.00	0.00	-8.33		
Min, Max	0.0, 66.7	-50.0, 16.7	0.0, 50.0	-66.7, 33.3		

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Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	10	10	27	27
Mean (SD)	13.73 (25.163)	-2.94 (12.127)	41.67 (41.759)	13.33 (46.348)	24.07 (34.385)	3.09 (29.970)
Median	0.00	0.00	25.00	0.00	0.00	0.00
Min, Max	0.0, 83.3	-33.3, 16.7	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0
Week 56						
n	12	12	10	9	22	21
Mean (SD)	22.22 (28.721)	1.39 (25.084)	36.67 (18.922)	9.26 (30.174)	28.79 (25.293)	4.76 (26.948)
Median	0.00	0.00	33.33	16.67	33.33	0.00
Min, Max	0.0, 66.7	-33.3, 66.7	16.7, 66.7	-33.3, 66.7	0.0, 66.7	-33.3, 66.7

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Table GERMANY.2.5.4.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Pain

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	7	7	8	7	15	14
Mean (SD)	2.38 (6.299)	-11.90 (15.853)	33.33 (32.121)	16.67 (40.825)	18.89 (28.078)	2.38 (33.242)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 16.7	-33.3, 0.0	0.0, 100.0	-33.3, 83.3	0.0, 100.0	-33.3, 83.3
Week 88						
n	5	5	5	5	10	10
Mean (SD)	6.67 (14.907)	-6.67 (9.129)	36.67 (32.059)	26.67 (38.370)	21.67 (28.382)	10.00 (31.623)
Median	0.00	0.00	33.33	33.33	8.33	0.00
Min, Max	0.0, 33.3	-16.7, 0.0	0.0, 83.3	-33.3, 66.7	0.0, 83.3	-33.3, 66.7

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.4.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Pain

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	16.67 (NA)	16.67 (NA)	8.33 (11.785)	8.33 (11.785)
Median	0.00	0.00	16.67	16.67	8.33	8.33
Min, Max	0.0, 0.0	0.0, 0.0	16.7, 16.7	16.7, 16.7	0.0, 16.7	0.0, 16.7

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Table GERMANY.2.5.4.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Dyspnoea

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		10		31	
Mean (SD)	36.51 (33.174)		26.67 (26.294)		33.33 (31.032)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	21	21	11	9		
Mean (SD)	30.16 (29.636)	-6.35 (22.655)	27.27 (25.025)	3.70 (30.932)		
Median	33.33	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	-33.3, 66.7		

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Dyspnoea

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	21	21	11	10		
Mean (SD)	20.63 (22.301)	-15.87 (29.096)	24.24 (33.635)	0.00 (31.427)		
Median	33.33	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		
Week 8						
n	14	14	6	5		
Mean (SD)	23.81 (20.375)	-9.52 (24.209)	44.44 (34.427)	13.33 (38.006)		
Median	33.33	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-33.3, 66.7		

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Dyspnoea

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	17	17	11	10		
Mean (SD)	21.57 (23.396)	-15.69 (26.661)	33.33 (25.820)	6.67 (40.976)		
Median	33.33	0.00	33.33	16.67		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-66.7, 66.7		
Week 24						
n	14	14	11	10		
Mean (SD)	19.05 (21.540)	-14.29 (28.388)	12.12 (16.817)	-13.33 (28.109)		
Median	16.67	-16.67	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 33.3	-66.7, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Dyspnoea

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	10	10	27	27
Mean (SD)	23.53 (25.725)	-21.57 (31.049)	13.33 (17.213)	-13.33 (28.109)	19.75 (23.130)	-18.52 (29.719)
Median	33.33	-33.33	0.00	0.00	33.33	-33.33
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 100.0	-66.7, 33.3
Week 56						
n	12	12	10	9	22	21
Mean (SD)	25.00 (25.126)	-25.00 (28.868)	13.33 (17.213)	-11.11 (28.868)	19.70 (22.204)	-19.05 (29.005)
Median	33.33	-33.33	0.00	0.00	16.67	-33.33
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-66.7, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Dyspnoea

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	7	7	8	7	15	14
Mean (SD)	19.05 (26.227)	-33.33 (19.245)	16.67 (25.198)	-14.29 (37.796)	17.78 (24.774)	-23.81 (30.462)
Median	0.00	-33.33	0.00	-33.33	0.00	-33.33
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-66.7, 33.3
Week 88						
n	5	5	5	5	10	10
Mean (SD)	20.00 (29.814)	-20.00 (18.257)	13.33 (18.257)	-13.33 (38.006)	16.67 (23.570)	-16.67 (28.328)
Median	0.00	-33.33	0.00	0.00	0.00	-16.67
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-66.7, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Dyspnoea

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	-33.33 (NA)	0.00 (NA)	0.00 (NA)	0.00 (0.000)	-16.67 (23.570)
Median	0.00	-33.33	0.00	0.00	0.00	-16.67
Min, Max	0.0, 0.0	-33.3, -33.3	0.0, 0.0	0.0, 0.0	0.0, 0.0	-33.3, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Insomnia

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		10		31	
Mean (SD)	30.16 (31.455)		36.67 (33.148)		32.26 (31.604)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	21	21	11	9		
Mean (SD)	23.81 (28.172)	-6.35 (20.053)	36.36 (27.707)	0.00 (44.096)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-66.7, 66.7		

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Insomnia

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	21	21	11	10		
Mean (SD)	30.16 (33.174)	0.00 (25.820)	30.30 (31.463)	-6.67 (46.614)		
Median	33.33	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 100.0	-100.0, 66.7		
Week 8						
n	14	14	6	5		
Mean (SD)	16.67 (21.681)	-9.52 (20.375)	11.11 (17.213)	-26.67 (43.461)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 33.3	-100.0, 0.0		

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Table GERMANY.2.5.4.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Insomnia

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	17	17	11	10		
Mean (SD)	23.53 (28.296)	-7.84 (18.743)	36.36 (37.873)	0.00 (49.690)		
Median	33.33	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 66.7		
Week 24						
n	14	14	11	10		
Mean (SD)	26.19 (32.499)	-2.38 (33.242)	21.21 (22.473)	-16.67 (32.394)		
Median	16.67	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 66.7	-66.7, 33.3		

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Insomnia

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	10	10	27	27
Mean (SD)	27.45 (29.428)	-7.84 (25.082)	33.33 (41.574)	-3.33 (53.171)	29.63 (33.758)	-6.17 (37.023)
Median	33.33	0.00	16.67	0.00	33.33	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-100.0, 66.7	0.0, 100.0	-100.0, 66.7
Week 56						
n	12	12	10	9	22	21
Mean (SD)	30.56 (30.011)	-5.56 (34.329)	43.33 (31.623)	3.70 (35.136)	36.36 (30.704)	-1.59 (34.118)
Median	33.33	-16.67	33.33	0.00	33.33	0.00
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 66.7

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Insomnia

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	7	7	8	7	15	14
Mean (SD)	14.29 (17.817)	-19.05 (17.817)	33.33 (30.861)	-4.76 (59.094)	24.44 (26.627)	-11.90 (42.582)
Median	0.00	-33.33	33.33	0.00	33.33	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-100.0, 66.7	0.0, 66.7	-100.0, 66.7
Week 88						
n	5	5	5	5	10	10
Mean (SD)	20.00 (29.814)	-13.33 (18.257)	40.00 (43.461)	6.67 (49.441)	30.00 (36.683)	-3.33 (36.683)
Median	0.00	0.00	33.33	0.00	16.67	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7

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Insomnia

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	33.33 (NA)	0.00 (NA)	100.00 (NA)	66.67 (NA)	66.67 (47.140)	33.33 (47.140)
Median	33.33	0.00	100.00	66.67	66.67	33.33
Min, Max	33.3, 33.3	0.0, 0.0	100.0, 100.0	66.7, 66.7	33.3, 100.0	0.0, 66.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Appetite Loss

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		10		31	
Mean (SD)	7.94 (14.548)		10.00 (16.102)		8.60 (14.827)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 33.3		0.0, 33.3		0.0, 33.3	
Week 2						
n	21	21	11	9		
Mean (SD)	12.70 (19.653)	4.76 (19.107)	18.18 (22.918)	7.41 (14.699)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	0.0, 33.3		

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Appetite Loss

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	21	21	11	10		
Mean (SD)	6.35 (13.412)	-1.59 (12.807)	12.12 (30.814)	3.33 (24.595)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 66.7		
Week 8						
n	14	14	6	5		
Mean (SD)	14.29 (21.540)	2.38 (27.625)	5.56 (13.608)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 33.3	0.0, 0.0		

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Appetite Loss

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	17	17	11	10		
Mean (SD)	13.73 (29.009)	7.84 (34.419)	12.12 (26.968)	3.33 (18.922)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 66.7	-33.3, 33.3		
Week 24						
n	14	14	11	10		
Mean (SD)	19.05 (28.388)	14.29 (31.254)	12.12 (16.817)	3.33 (10.541)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 33.3	0.0, 33.3		

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Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	10	10	27	27
Mean (SD)	9.80 (15.656)	0.00 (20.412)	23.33 (35.312)	13.33 (35.832)	14.81 (25.036)	4.94 (27.275)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0
Week 56						
n	12	12	10	9	22	21
Mean (SD)	11.11 (16.412)	2.78 (22.285)	6.67 (14.055)	-3.70 (11.111)	9.09 (15.195)	0.00 (18.257)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 33.3

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Appetite Loss

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	7	7	8	7	15	14
Mean (SD)	0.00 (0.000)	-9.52 (16.265)	16.67 (25.198)	9.52 (25.198)	8.89 (19.787)	0.00 (22.646)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 66.7	0.0, 66.7	0.0, 66.7	-33.3, 66.7
Week 88						
n	5	5	5	5	10	10
Mean (SD)	20.00 (18.257)	6.67 (14.907)	20.00 (18.257)	13.33 (18.257)	20.00 (17.213)	10.00 (16.102)
Median	33.33	0.00	33.33	0.00	33.33	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3

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Table GERMANY.2.5.4.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Appetite Loss

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	66.67 (NA)	33.33 (NA)	33.33 (47.140)	16.67 (23.570)
Median	0.00	0.00	66.67	33.33	33.33	16.67
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	33.3, 33.3	0.0, 66.7	0.0, 33.3

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.4.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Constipation

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		10		31	
Mean (SD)	11.11 (21.943)		13.33 (17.213)		11.83 (20.273)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 33.3		0.0, 66.7	
Week 2						
n	21	21	11	9		
Mean (SD)	6.35 (17.059)	-4.76 (19.107)	18.18 (31.140)	3.70 (30.932)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Constipation

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	21	21	11	10		
Mean (SD)	4.76 (15.936)	-6.35 (20.053)	24.24 (30.151)	13.33 (23.307)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	0.0, 66.7		
Week 8						
n	14	14	6	5		
Mean (SD)	0.00 (0.000)	-9.52 (20.375)	22.22 (27.217)	13.33 (18.257)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 66.7	0.0, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Constipation

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	17	17	11	10		
Mean (SD)	0.00 (0.000)	-9.80 (19.596)	21.21 (34.230)	10.00 (22.498)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 100.0	0.0, 66.7		
Week 24						
n	14	14	11	10		
Mean (SD)	0.00 (0.000)	-7.14 (19.298)	15.15 (22.918)	3.33 (18.922)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 66.7	-33.3, 33.3		

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Constipation

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	10	10	27	27
Mean (SD)	1.96 (8.085)	-11.76 (20.211)	26.67 (26.294)	13.33 (23.307)	11.11 (20.672)	-2.47 (24.330)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 66.7	0.0, 66.7	0.0, 66.7	-66.7, 66.7
Week 56						
n	12	12	10	9	22	21
Mean (SD)	5.56 (12.975)	-8.33 (25.126)	16.67 (23.570)	0.00 (16.667)	10.61 (18.930)	-4.76 (21.822)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-66.7, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Constipation

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	7	7	8	7	15	14
Mean (SD)	4.76 (12.599)	-9.52 (16.265)	12.50 (17.252)	4.76 (23.002)	8.89 (15.258)	-2.38 (20.524)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3
Week 88						
n	5	5	5	5	10	10
Mean (SD)	13.33 (18.257)	0.00 (23.570)	40.00 (54.772)	33.33 (47.140)	26.67 (40.976)	16.67 (39.284)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	0.0, 100.0	0.0, 100.0	-33.3, 100.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Constipation

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	66.67 (NA)	33.33 (NA)	33.33 (47.140)	16.67 (23.570)
Median	0.00	0.00	66.67	33.33	33.33	16.67
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	33.3, 33.3	0.0, 66.7	0.0, 33.3

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Table GERMANY.2.5.4.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Diarrhea

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		10		31	
Mean (SD)	4.76 (11.952)		20.00 (28.109)		9.68 (19.614)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 33.3		0.0, 66.7		0.0, 66.7	
Week 2						
n	21	21	11	9		
Mean (SD)	3.17 (10.026)	-1.59 (7.274)	3.03 (10.050)	-18.52 (24.216)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-66.7, 0.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Diarrhea

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	21	21	11	10		
Mean (SD)	4.76 (11.952)	0.00 (14.907)	3.03 (10.050)	-16.67 (23.570)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-66.7, 0.0		
Week 8						
n	14	14	6	5		
Mean (SD)	14.29 (21.540)	11.90 (21.111)	16.67 (27.889)	-13.33 (18.257)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	0.0, 66.7	0.0, 66.7	-33.3, 0.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Diarrhea

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	17	17	11	10		
Mean (SD)	5.88 (13.098)	0.00 (11.785)	6.06 (20.101)	-13.33 (39.126)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-66.7, 66.7		
Week 24						
n	14	14	11	10		
Mean (SD)	4.76 (12.105)	2.38 (8.909)	6.06 (13.484)	-13.33 (23.307)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	-66.7, 0.0		

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Table GERMANY.2.5.4.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Diarrhea

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	10	10	27	27
Mean (SD)	7.84 (14.575)	1.96 (18.524)	6.67 (14.055)	-13.33 (28.109)	7.41 (14.122)	-3.70 (23.266)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3
Week 56						
n	12	12	10	9	22	21
Mean (SD)	8.33 (15.076)	5.56 (12.975)	10.00 (16.102)	-14.81 (29.397)	9.09 (15.195)	-3.17 (23.345)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3

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Table GERMANY.2.5.4.7

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Diarrhea

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	7	7	8	7	15	14
Mean (SD)	0.00 (0.000)	-4.76 (12.599)	33.33 (35.635)	14.29 (42.414)	17.78 (30.516)	4.76 (31.642)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0
Week 88						
n	5	5	5	5	10	10
Mean (SD)	0.00 (0.000)	0.00 (0.000)	6.67 (14.907)	-6.67 (36.515)	3.33 (10.541)	-3.33 (24.595)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3

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Diarrhea

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	0.00 (NA)	0.00 (NA)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0

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Financial Difficulties

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		10		31	
Mean (SD)	22.22 (33.884)		26.67 (34.427)		23.66 (33.548)	
Median	0.00		16.67		0.00	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	21	21	11	9		
Mean (SD)	20.63 (32.449)	-1.59 (12.807)	24.24 (39.696)	3.70 (42.310)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 100.0		

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Financial Difficulties

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	21	21	11	10		
Mean (SD)	22.22 (32.203)	0.00 (14.907)	18.18 (40.452)	-6.67 (43.885)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 100.0		
Week 8						
n	14	14	6	5		
Mean (SD)	4.76 (12.105)	-9.52 (20.375)	5.56 (13.608)	-6.67 (14.907)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-33.3, 0.0		

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Financial Difficulties

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	17	17	11	10		
Mean (SD)	21.57 (31.049)	-1.96 (14.292)	21.21 (40.202)	-3.33 (42.889)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 100.0		
Week 24						
n	14	14	11	10		
Mean (SD)	19.05 (33.878)	-4.76 (28.815)	21.21 (40.202)	-3.33 (42.889)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 100.0	-66.7, 100.0		

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Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	10	10	27	27
Mean (SD)	21.57 (31.049)	-3.92 (23.221)	20.00 (35.832)	-6.67 (51.640)	20.99 (32.223)	-4.94 (35.450)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-100.0, 100.0	0.0, 100.0	-100.0, 100.0
Week 56						
n	12	12	10	9	22	21
Mean (SD)	22.22 (32.824)	-8.33 (15.076)	30.00 (39.907)	3.70 (45.474)	25.76 (35.533)	-3.17 (31.455)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 100.0	-33.3, 0.0	0.0, 100.0	-66.7, 100.0	0.0, 100.0	-66.7, 100.0

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Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	7	7	8	7	15	14
Mean (SD)	9.52 (16.265)	-14.29 (37.796)	8.33 (15.430)	-14.29 (26.227)	8.89 (15.258)	-14.29 (31.254)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 33.3
Week 88						
n	5	5	5	5	10	10
Mean (SD)	13.33 (18.257)	-13.33 (18.257)	6.67 (14.907)	-20.00 (29.814)	10.00 (16.102)	-16.67 (23.570)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0

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Financial Difficulties

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	-66.67 (NA)	0.00 (NA)	-66.67 (NA)	0.00 (0.000)	-66.67 (0.000)
Median	0.00	-66.67	0.00	-66.67	0.00	-66.67
Min, Max	0.0, 0.0	-66.7, -66.7	0.0, 0.0	-66.7, -66.7	0.0, 0.0	-66.7, -66.7

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Table GERMANY.2.5.4.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Fatigue

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		14		50	
Mean (SD)	45.99 (29.114)		52.38 (30.948)		47.78 (29.461)	
Median	44.44		50.00		44.44	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	34	34	14	14		
Mean (SD)	31.05 (25.773)	-15.69 (21.032)	46.03 (26.815)	-6.35 (22.525)		
Median	33.33	-11.11	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	11.1, 100.0	-44.4, 33.3		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Fatigue

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	36	36	14	14		
Mean (SD)	27.01 (22.816)	-18.98 (23.622)	44.44 (21.350)	-7.94 (24.818)		
Median	22.22	-11.11	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	11.1, 88.9	-66.7, 22.2		
Week 8						
n	31	31	13	13		
Mean (SD)	30.11 (23.517)	-15.77 (25.943)	34.19 (19.490)	-14.53 (24.167)		
Median	33.33	-11.11	33.33	-11.11		
Min, Max	0.0, 100.0	-66.7, 44.4	0.0, 66.7	-77.8, 11.1		

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Fatigue

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	35	35	14	14		
Mean (SD)	26.35 (20.182)	-19.37 (21.278)	38.10 (24.543)	-14.29 (25.940)		
Median	33.33	-11.11	33.33	-11.11		
Min, Max	0.0, 66.7	-66.7, 11.1	0.0, 100.0	-77.8, 22.2		
Week 24						
n	35	35	13	13		
Mean (SD)	23.17 (20.939)	-22.54 (25.776)	32.48 (26.239)	-16.24 (21.089)		
Median	22.22	-22.22	33.33	-22.22		
Min, Max	0.0, 100.0	-77.8, 33.3	0.0, 88.9	-44.4, 22.2		

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Fatigue

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	11	11	40	40
Mean (SD)	22.61 (22.709)	-19.16 (25.699)	39.39 (24.018)	-8.08 (19.933)	27.22 (23.996)	-16.11 (24.518)
Median	22.22	-11.11	33.33	0.00	22.22	-11.11
Min, Max	0.0, 100.0	-77.8, 33.3	0.0, 77.8	-55.6, 11.1	0.0, 100.0	-77.8, 33.3
Week 56						
n	22	22	7	7	29	29
Mean (SD)	28.28 (23.689)	-13.64 (27.843)	38.10 (16.798)	-11.11 (30.089)	30.65 (22.352)	-13.03 (27.868)
Median	33.33	-5.56	33.33	-11.11	33.33	-11.11
Min, Max	0.0, 100.0	-66.7, 33.3	22.2, 66.7	-66.7, 33.3	0.0, 100.0	-66.7, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Fatigue

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	14	14	4	4	18	18
Mean (SD)	28.57 (18.853)	-20.63 (23.814)	61.11 (11.111)	-11.11 (15.713)	35.80 (22.077)	-18.52 (22.222)
Median	27.78	-11.11	66.67	-5.56	33.33	-11.11
Min, Max	0.0, 66.7	-66.7, 22.2	44.4, 66.7	-33.3, 0.0	0.0, 66.7	-66.7, 22.2
Week 88						
n	9	9	3	3	12	12
Mean (SD)	16.05 (18.519)	-29.63 (21.517)	44.44 (11.111)	-33.33 (29.397)	23.15 (20.899)	-30.56 (22.285)
Median	11.11	-33.33	44.44	-22.22	16.67	-27.78
Min, Max	0.0, 55.6	-66.7, 0.0	33.3, 55.6	-66.7, -11.1	0.0, 55.6	-66.7, 0.0

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Fatigue

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	18.52 (16.973)	-22.22 (29.397)	22.22 (NA)	-77.78 (NA)	19.44 (13.981)	-36.11 (36.712)
Median	22.22	-11.11	22.22	-77.78	22.22	-33.33
Min, Max	0.0, 33.3	-55.6, 0.0	22.2, 22.2	-77.8, -77.8	0.0, 33.3	-77.8, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Nausea and Vomiting

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		14		50	
Mean (SD)	3.70 (12.669)		9.52 (19.298)		5.33 (14.846)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	34	34	14	14		
Mean (SD)	4.41 (12.519)	0.49 (16.659)	8.33 (15.676)	-1.19 (10.262)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 50.0	-50.0, 50.0	0.0, 50.0	-16.7, 16.7		

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Nausea and Vomiting

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	36	36	14	14		
Mean (SD)	4.17 (10.059)	0.46 (14.078)	4.76 (10.187)	-4.76 (21.111)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-50.0, 33.3	0.0, 33.3	-66.7, 16.7		
Week 8						
n	31	31	13	13		
Mean (SD)	3.76 (9.339)	-0.54 (11.772)	5.13 (14.248)	-2.56 (21.350)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 50.0	-66.7, 33.3		

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Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	35	35	14	14		
Mean (SD)	4.29 (10.180)	0.48 (12.450)	8.33 (22.408)	-1.19 (23.987)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-50.0, 33.3	0.0, 83.3	-66.7, 50.0		
Week 24						
n	35	35	13	13		
Mean (SD)	2.86 (11.775)	-0.95 (15.094)	1.28 (4.623)	-3.85 (12.084)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-50.0, 66.7	0.0, 16.7	-33.3, 16.7		

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Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	11	11	40	40
Mean (SD)	3.45 (6.871)	1.72 (6.821)	3.03 (6.742)	-1.52 (8.989)	3.33 (6.752)	0.83 (7.501)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	-16.7, 16.7	0.0, 16.7	-16.7, 16.7	0.0, 16.7	-16.7, 16.7
Week 56						
n	22	22	7	7	29	29
Mean (SD)	0.00 (0.000)	-2.27 (7.793)	4.76 (8.133)	-2.38 (14.996)	1.15 (4.298)	-2.30 (9.682)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 16.7	-33.3, 16.7	0.0, 16.7	-33.3, 16.7

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Table GERMANY.2.5.4.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Nausea and Vomiting

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	14	14	4	4	18	18
Mean (SD)	1.19 (4.454)	0.00 (6.537)	0.00 (0.000)	-12.50 (15.957)	0.93 (3.928)	-2.78 (10.306)
Median	0.00	0.00	0.00	-8.33	0.00	0.00
Min, Max	0.0, 16.7	-16.7, 16.7	0.0, 0.0	-33.3, 0.0	0.0, 16.7	-33.3, 16.7
Week 88						
n	9	9	3	3	12	12
Mean (SD)	0.00 (0.000)	0.00 (0.000)	11.11 (9.623)	-5.56 (25.459)	2.78 (6.487)	-1.39 (11.143)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 16.7	-33.3, 16.7	0.0, 16.7	-33.3, 16.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Nausea and Vomiting

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	0.00 (0.000)	0.00 (0.000)	66.67 (NA)	33.33 (NA)	16.67 (33.333)	8.33 (16.667)
Median	0.00	0.00	66.67	33.33	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	33.3, 33.3	0.0, 66.7	0.0, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Pain

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		14		50	
Mean (SD)	15.28 (26.540)		15.48 (21.147)		15.33 (24.935)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	34	34	14	14		
Mean (SD)	13.24 (23.847)	-2.94 (21.505)	20.24 (20.856)	4.76 (12.105)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 66.7	-16.7, 33.3		

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Pain

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	36	36	14	14		
Mean (SD)	11.11 (21.455)	-4.17 (16.607)	20.24 (30.786)	4.76 (24.832)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 16.7	0.0, 100.0	-33.3, 83.3		
Week 8						
n	30	30	13	13		
Mean (SD)	13.89 (24.006)	1.67 (25.652)	10.26 (14.495)	-5.13 (17.193)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 33.3	-33.3, 16.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Pain

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	35	35	14	14		
Mean (SD)	12.38 (23.689)	-3.33 (23.501)	23.81 (27.514)	8.33 (26.755)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-83.3, 33.3	0.0, 100.0	-16.7, 83.3		
Week 24						
n	35	35	13	13		
Mean (SD)	12.38 (25.355)	-3.33 (26.134)	15.38 (24.019)	1.28 (12.659)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-83.3, 66.7	0.0, 83.3	-33.3, 16.7		

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Pain

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	11	11	40	40
Mean (SD)	12.64 (22.997)	-1.72 (23.715)	10.61 (15.407)	3.03 (12.513)	12.08 (21.010)	-0.42 (21.179)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 83.3	-83.3, 33.3	0.0, 33.3	-16.7, 33.3	0.0, 83.3	-83.3, 33.3
Week 56						
n	22	22	7	7	29	29
Mean (SD)	14.39 (25.869)	-3.79 (26.192)	9.52 (18.898)	2.38 (24.398)	13.22 (24.144)	-2.30 (25.481)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-83.3, 33.3	0.0, 50.0	-33.3, 50.0	0.0, 100.0	-83.3, 50.0

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Pain

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	4	4	17	17
Mean (SD)	11.54 (19.702)	-7.69 (22.169)	0.00 (0.000)	-4.17 (8.333)	8.82 (17.793)	-6.86 (19.596)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-50.0, 16.7	0.0, 0.0	-16.7, 0.0	0.0, 66.7	-50.0, 16.7
Week 88						
n	9	9	3	3	12	12
Mean (SD)	11.11 (22.048)	-9.26 (25.154)	5.56 (9.623)	0.00 (0.000)	9.72 (19.408)	-6.94 (21.856)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 16.7	0.0, 16.7	0.0, 0.0	0.0, 66.7	-66.7, 16.7

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Pain

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	11.11 (19.245)	-22.22 (38.490)	16.67 (NA)	0.00 (NA)	12.50 (15.957)	-16.67 (33.333)
Median	0.00	0.00	16.67	0.00	8.33	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	16.7, 16.7	0.0, 0.0	0.0, 33.3	-66.7, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Dyspnoea

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		14		50	
Mean (SD)	37.04 (26.159)		33.33 (32.026)		36.00 (27.635)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	35	35	14	14		
Mean (SD)	18.10 (23.351)	-20.00 (21.693)	38.10 (28.815)	4.76 (25.678)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 33.3		

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Table GERMANY.2.5.4.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Dyspnoea

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	36	36	14	14		
Mean (SD)	11.11 (22.537)	-25.93 (22.693)	40.48 (32.499)	7.14 (35.030)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 66.7		
Week 8						
n	31	31	13	13		
Mean (SD)	16.13 (24.146)	-21.51 (23.646)	20.51 (21.681)	-12.82 (32.026)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 66.7	-100.0, 33.3		

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Dyspnoea

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	35	35	14	14		
Mean (SD)	14.29 (20.269)	-21.90 (24.176)	26.19 (23.310)	-7.14 (39.610)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-100.0, 66.7		
Week 24						
n	35	35	13	13		
Mean (SD)	15.24 (23.351)	-20.95 (26.918)	17.95 (17.296)	-10.26 (25.036)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 33.3	-66.7, 33.3		

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Dyspnoea

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	11	11	40	40
Mean (SD)	13.79 (22.743)	-18.39 (19.078)	18.18 (17.408)	-12.12 (16.817)	15.00 (21.284)	-16.67 (18.490)
Median	0.00	-33.33	33.33	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-66.7, 0.0
Week 56						
n	22	22	7	7	29	29
Mean (SD)	21.21 (30.071)	-7.58 (22.845)	9.52 (16.265)	-4.76 (23.002)	18.39 (27.583)	-6.90 (22.501)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-66.7, 33.3

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Dyspnoea

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	14	14	4	4	18	18
Mean (SD)	11.90 (21.111)	-23.81 (20.375)	25.00 (31.914)	0.00 (27.217)	14.81 (23.493)	-18.52 (23.493)
Median	0.00	-33.33	16.67	0.00	0.00	-33.33
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-66.7, 33.3
Week 88						
n	9	9	3	3	12	12
Mean (SD)	0.00 (0.000)	-29.63 (26.058)	0.00 (0.000)	-22.22 (19.245)	0.00 (0.000)	-27.78 (23.925)
Median	0.00	-33.33	0.00	-33.33	0.00	-33.33
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 0.0	-33.3, 0.0	0.0, 0.0	-66.7, 0.0

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Dyspnoea

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	0.00 (0.000)	-22.22 (38.490)	33.33 (NA)	0.00 (NA)	8.33 (16.667)	-16.67 (33.333)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 0.0	-66.7, 0.0	33.3, 33.3	0.0, 0.0	0.0, 33.3	-66.7, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Insomnia

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		14		50	
Mean (SD)	24.07 (25.956)		23.81 (30.462)		24.00 (26.971)	
Median	33.33		16.67		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	34	34	14	14		
Mean (SD)	20.59 (31.798)	-3.92 (19.703)	23.81 (35.635)	0.00 (22.646)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 33.3		

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Insomnia

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	36	36	14	14		
Mean (SD)	15.74 (29.262)	-8.33 (23.060)	21.43 (30.959)	-2.38 (27.625)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 33.3		
Week 8						
n	31	31	13	13		
Mean (SD)	11.83 (18.355)	-13.98 (26.908)	17.95 (29.235)	0.00 (23.570)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-33.3, 33.3		

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Insomnia

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Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	35	35	14	14		
Mean (SD)	13.33 (21.693)	-10.48 (21.038)	26.19 (29.753)	2.38 (20.524)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-33.3, 33.3		
Week 24						
n	35	35	13	13		
Mean (SD)	10.48 (21.038)	-13.33 (25.820)	20.51 (25.598)	-2.56 (16.452)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 66.7	-33.3, 33.3		

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Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	11	11	40	40
Mean (SD)	16.09 (26.157)	-5.75 (23.686)	18.18 (22.918)	-6.06 (25.025)	16.67 (25.036)	-5.83 (23.736)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-66.7, 33.3
Week 56						
n	22	22	7	7	29	29
Mean (SD)	16.67 (24.667)	-3.03 (20.339)	28.57 (35.635)	0.00 (33.333)	19.54 (27.483)	-2.30 (23.454)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 33.3

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Insomnia

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	14	14	4	4	18	18
Mean (SD)	11.90 (21.111)	-9.52 (20.375)	50.00 (43.033)	0.00 (27.217)	20.37 (30.548)	-7.41 (21.559)
Median	0.00	0.00	50.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 33.3
Week 88						
n	9	9	3	3	12	12
Mean (SD)	3.70 (11.111)	-11.11 (16.667)	44.44 (50.918)	-11.11 (50.918)	13.89 (30.011)	-11.11 (25.950)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Insomnia

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	0.00 (0.000)	-22.22 (38.490)	0.00 (NA)	-100.00 (NA)	0.00 (0.000)	-41.67 (50.000)
Median	0.00	0.00	0.00	-100.00	0.00	-33.33
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 0.0	-100.0, -100.0	0.0, 0.0	-100.0, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Appetite Loss

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		14		50	
Mean (SD)	9.26 (17.110)		23.81 (33.150)		13.33 (23.328)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 100.0		0.0, 100.0	
Week 2						
n	35	35	14	14		
Mean (SD)	8.57 (18.687)	-0.95 (18.935)	21.43 (24.832)	-2.38 (15.821)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-33.3, 33.3		

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Appetite Loss

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	36	36	14	14		
Mean (SD)	5.56 (14.907)	-3.70 (15.487)	14.29 (25.198)	-9.52 (27.514)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-66.7, 33.3		
Week 8						
n	31	31	13	13		
Mean (SD)	12.90 (18.614)	2.15 (17.074)	15.38 (22.008)	-5.13 (32.903)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-100.0, 33.3		

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Appetite Loss

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	35	35	14	14		
Mean (SD)	3.81 (10.760)	-5.71 (17.123)	21.43 (30.959)	-2.38 (27.625)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-66.7, 33.3		
Week 24						
n	35	35	13	13		
Mean (SD)	7.62 (19.944)	-1.90 (26.744)	20.51 (28.991)	2.56 (16.452)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 100.0	0.0, 66.7	-33.3, 33.3		

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Appetite Loss

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	11	11	40	40
Mean (SD)	4.60 (11.698)	-1.15 (14.037)	15.15 (17.408)	0.00 (14.907)	7.50 (14.097)	-0.83 (14.097)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3
Week 56						
n	22	22	7	7	29	29
Mean (SD)	6.06 (13.159)	1.52 (19.182)	19.05 (37.796)	0.00 (38.490)	9.20 (21.633)	1.15 (24.369)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Appetite Loss

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	14	14	4	4	18	18
Mean (SD)	4.76 (12.105)	-2.38 (8.909)	25.00 (16.667)	-8.33 (16.667)	9.26 (15.363)	-3.70 (10.779)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 0.0
Week 88						
n	9	9	3	3	12	12
Mean (SD)	0.00 (0.000)	-3.70 (11.111)	11.11 (19.245)	-22.22 (38.490)	2.78 (9.623)	-8.33 (20.719)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Appetite Loss

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	0.00 (0.000)	0.00 (0.000)	66.67 (NA)	0.00 (NA)	16.67 (33.333)	0.00 (0.000)
Median	0.00	0.00	66.67	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	0.0, 0.0	0.0, 66.7	0.0, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Constipation

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		14		50	
Mean (SD)	14.81 (24.488)		9.52 (20.375)		13.33 (23.328)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	34	34	14	14		
Mean (SD)	11.76 (25.797)	-3.92 (19.703)	16.67 (31.351)	7.14 (23.310)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

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Constipation

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	36	36	14	14		
Mean (SD)	12.96 (25.545)	-1.85 (22.459)	14.29 (28.388)	4.76 (25.678)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-33.3, 66.7		
Week 8						
n	31	31	13	13		
Mean (SD)	8.60 (17.144)	-5.38 (21.251)	12.82 (32.026)	5.13 (12.518)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	0.0, 33.3		

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Table GERMANY.2.5.4.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Constipation

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	35	35	14	14		
Mean (SD)	14.29 (28.337)	-0.95 (20.589)	14.29 (31.254)	4.76 (22.100)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 66.7		
Week 24						
n	35	35	13	13		
Mean (SD)	9.52 (22.246)	-5.71 (20.589)	17.95 (29.235)	7.69 (24.167)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 66.7		

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Constipation

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	11	11	40	40
Mean (SD)	9.20 (17.586)	-6.90 (22.501)	6.06 (13.484)	-3.03 (17.979)	8.33 (16.452)	-5.83 (21.200)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-66.7, 33.3
Week 56						
n	22	22	7	7	29	29
Mean (SD)	12.12 (26.318)	-7.58 (17.614)	4.76 (12.599)	0.00 (0.000)	10.34 (23.744)	-5.75 (15.607)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 0.0	0.0, 33.3	0.0, 0.0	0.0, 100.0	-66.7, 0.0

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Constipation

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	14	14	4	4	18	18
Mean (SD)	16.67 (28.495)	-7.14 (23.310)	8.33 (16.667)	0.00 (0.000)	14.81 (26.127)	-5.56 (20.612)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 33.3	0.0, 0.0	0.0, 100.0	-33.3, 33.3
Week 88						
n	9	9	3	3	12	12
Mean (SD)	14.81 (17.568)	-7.41 (22.222)	0.00 (0.000)	-11.11 (19.245)	11.11 (16.412)	-8.33 (20.719)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 0.0	-33.3, 0.0	0.0, 33.3	-33.3, 33.3

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Constipation

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	22.22 (19.245)	-11.11 (19.245)	0.00 (NA)	-33.33 (NA)	16.67 (19.245)	-16.67 (19.245)
Median	33.33	0.00	0.00	-33.33	16.67	-16.67
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 0.0	-33.3, -33.3	0.0, 33.3	-33.3, 0.0

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Diarrhea

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		14		50	
Mean (SD)	5.56 (18.687)		7.14 (14.194)		6.00 (17.418)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 33.3		0.0, 100.0	
Week 2						
n	35	35	14	14		
Mean (SD)	7.62 (21.520)	1.90 (21.302)	7.14 (19.298)	0.00 (18.490)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 100.0	0.0, 66.7	-33.3, 33.3		

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Diarrhea

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	36	36	14	14		
Mean (SD)	5.56 (14.907)	0.00 (22.537)	4.76 (12.105)	-2.38 (15.821)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-100.0, 66.7	0.0, 33.3	-33.3, 33.3		
Week 8						
n	31	31	13	13		
Mean (SD)	4.30 (11.359)	-2.15 (17.074)	2.56 (9.245)	-5.13 (18.490)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-33.3, 33.3		

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Diarrhea

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	35	35	14	14		
Mean (SD)	5.71 (12.746)	0.00 (18.078)	2.38 (8.909)	-4.76 (17.817)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-33.3, 33.3		
Week 24						
n	35	35	13	13		
Mean (SD)	5.71 (12.746)	0.00 (18.078)	2.56 (9.245)	-2.56 (9.245)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-33.3, 0.0		

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Diarrhea

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	11	11	40	40
Mean (SD)	3.45 (10.331)	-3.45 (22.440)	9.09 (15.570)	3.03 (17.979)	5.00 (12.054)	-1.67 (21.284)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-100.0, 33.3
Week 56						
n	22	22	7	7	29	29
Mean (SD)	6.06 (13.159)	0.00 (25.198)	4.76 (12.599)	-4.76 (12.599)	5.75 (12.814)	-1.15 (22.683)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-100.0, 33.3

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Diarrhea

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	4	4	17	17
Mean (SD)	0.00 (0.000)	0.00 (0.000)	8.33 (16.667)	-8.33 (16.667)	1.96 (8.085)	-1.96 (8.085)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 0.0
Week 88						
n	9	9	3	3	12	12
Mean (SD)	3.70 (11.111)	3.70 (11.111)	11.11 (19.245)	-11.11 (19.245)	5.56 (12.975)	0.00 (14.213)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 33.3

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Diarrhea

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (NA)	0.00 (NA)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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Table GERMANY.2.5.4.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Financial Difficulties

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		14		50	
Mean (SD)	19.44 (29.141)		21.43 (30.959)		20.00 (29.354)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	36	36	14	14		
Mean (SD)	16.67 (28.172)	-2.78 (16.667)	23.81 (30.462)	2.38 (15.821)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 33.3		

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Financial Difficulties

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	36	36	14	14		
Mean (SD)	14.81 (23.155)	-4.63 (22.752)	19.05 (28.388)	-2.38 (15.821)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-33.3, 33.3		
Week 8						
n	31	31	13	13		
Mean (SD)	16.13 (24.146)	-5.38 (22.928)	17.95 (25.875)	2.56 (21.350)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 66.7	-33.3, 33.3		

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Financial Difficulties

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	35	35	14	14		
Mean (SD)	16.19 (24.749)	-2.86 (18.737)	23.81 (33.150)	2.38 (24.335)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		
Week 24						
n	35	35	13	13		
Mean (SD)	17.14 (27.262)	-1.90 (24.176)	23.08 (31.578)	0.00 (13.608)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 100.0	0.0, 100.0	-33.3, 33.3		

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Financial Difficulties

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	11	11	40	40
Mean (SD)	14.94 (24.537)	-5.75 (23.686)	21.21 (30.814)	-6.06 (13.484)	16.67 (26.149)	-5.83 (21.200)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 0.0	0.0, 100.0	-66.7, 33.3
Week 56						
n	22	22	7	7	29	29
Mean (SD)	7.58 (14.298)	-9.09 (21.036)	19.05 (26.227)	-9.52 (31.706)	10.34 (18.046)	-9.20 (23.395)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-66.7, 33.3

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Table GERMANY.2.5.4.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Financial Difficulties

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	4	4	17	17
Mean (SD)	7.69 (14.618)	-7.69 (14.618)	16.67 (33.333)	-16.67 (19.245)	9.80 (19.596)	-9.80 (15.656)
Median	0.00	0.00	0.00	-16.67	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-33.3, 0.0	0.0, 66.7	-33.3, 0.0
Week 88						
n	9	9	3	3	12	12
Mean (SD)	7.41 (14.699)	-7.41 (22.222)	11.11 (19.245)	-33.33 (33.333)	8.33 (15.076)	-13.89 (26.432)
Median	0.00	0.00	0.00	-33.33	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0

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Table GERMANY.2.5.4.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Financial Difficulties

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	0.00 (0.000)	0.00 (0.000)	33.33 (NA)	-66.67 (NA)	8.33 (16.667)	-16.67 (33.333)
Median	0.00	0.00	33.33	-66.67	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	33.3, 33.3	-66.7, -66.7	0.0, 33.3	-66.7, 0.0

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Table GERMANY.2.5.4.8

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Fatigue

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	43.06 (23.335)		27.78 (11.111)		37.96 (20.899)	
Median	38.89		22.22		27.78	
Min, Max	22.2, 88.9		22.2, 44.4		22.2, 88.9	
Week 2						
n	8	8	4	4		
Mean (SD)	36.11 (22.023)	-6.94 (16.733)	33.33 (0.000)	5.56 (11.111)		
Median	44.44	-5.56	33.33	11.11		
Min, Max	0.0, 55.6	-33.3, 22.2	33.3, 33.3	-11.1, 11.1		

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Modified Randomized Set

Fatigue

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	8	8	4	4		
Mean (SD)	33.33 (25.198)	-9.72 (23.335)	36.11 (5.556)	8.33 (13.981)		
Median	38.89	-11.11	33.33	11.11		
Min, Max	0.0, 77.8	-55.6, 22.2	33.3, 44.4	-11.1, 22.2		
Week 8						
n	8	8	4	4		
Mean (SD)	37.50 (22.954)	-5.56 (19.698)	27.78 (6.415)	0.00 (15.713)		
Median	38.89	-5.56	27.78	5.56		
Min, Max	11.1, 77.8	-44.4, 22.2	22.2, 33.3	-22.2, 11.1		

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Fatigue

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	8	8	4	4		
Mean (SD)	31.94 (22.567)	-11.11 (23.757)	33.33 (9.072)	5.56 (14.344)		
Median	33.33	-11.11	33.33	5.56		
Min, Max	0.0, 55.6	-55.6, 22.2	22.2, 44.4	-11.1, 22.2		
Week 24						
n	8	8	4	4		
Mean (SD)	26.39 (22.173)	-16.67 (24.488)	33.33 (12.830)	5.56 (11.111)		
Median	33.33	-22.22	33.33	0.00		
Min, Max	0.0, 66.7	-55.6, 33.3	22.2, 44.4	0.0, 22.2		

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Table GERMANY.2.5.4.8

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Fatigue

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	18.52 (18.144)	-20.37 (30.965)	29.63 (6.415)	0.00 (11.111)	22.22 (15.713)	-13.58 (27.090)
Median	16.67	-16.67	33.33	0.00	22.22	-11.11
Min, Max	0.0, 44.4	-77.8, 11.1	22.2, 33.3	-11.1, 11.1	0.0, 44.4	-77.8, 11.1
Week 56						
n	4	4	1	1	5	5
Mean (SD)	36.11 (35.573)	5.56 (26.450)	22.22 (NA)	0.00 (NA)	33.33 (31.427)	4.44 (23.040)
Median	38.89	5.56	22.22	0.00	22.22	0.00
Min, Max	0.0, 66.7	-22.2, 33.3	22.2, 22.2	0.0, 0.0	0.0, 66.7	-22.2, 33.3

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.4.8

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Fatigue

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	1	1	0	0	1	1
Mean (SD)	33.33 (NA)	-11.11 (NA)			33.33 (NA)	-11.11 (NA)
Median	33.33	-11.11			33.33	-11.11
Min, Max	33.3, 33.3	-11.1, -11.1			33.3, 33.3	-11.1, -11.1

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Table GERMANY.2.5.4.8

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Nausea and Vomiting

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	0.00 (0.000)		0.00 (0.000)		0.00 (0.000)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 0.0		0.0, 0.0		0.0, 0.0	
Week 2						
n	8	8	4	4		
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0		

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Modified Randomized Set

Nausea and Vomiting

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	8	8	4	4		
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0		
Week 8						
n	8	8	4	4		
Mean (SD)	2.08 (5.893)	2.08 (5.893)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 16.7	0.0, 16.7	0.0, 0.0	0.0, 0.0		

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Nausea and Vomiting

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	8	8	4	4		
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0		
Week 24						
n	8	8	4	4		
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0		

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Modified Randomized Set

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Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0
Week 56						
n	4	4	1	1	5	5
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (NA)	0.00 (NA)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0

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Modified Randomized Set

Nausea and Vomiting

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	0.00 (NA)			0.00 (NA)	0.00 (NA)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Modified Randomized Set

Pain

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	0.00 (0.000)		16.67 (19.245)		5.56 (12.975)	
Median	0.00		16.67		0.00	
Min, Max	0.0, 0.0		0.0, 33.3		0.0, 33.3	
Week 2						
n	8	8	4	4		
Mean (SD)	2.08 (5.893)	2.08 (5.893)	20.83 (8.333)	4.17 (15.957)		
Median	0.00	0.00	16.67	8.33		
Min, Max	0.0, 16.7	0.0, 16.7	16.7, 33.3	-16.7, 16.7		

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Modified Randomized Set

Pain

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	8	8	4	4		
Mean (SD)	6.25 (17.678)	6.25 (17.678)	20.83 (15.957)	4.17 (8.333)		
Median	0.00	0.00	25.00	0.00		
Min, Max	0.0, 50.0	0.0, 50.0	0.0, 33.3	0.0, 16.7		
Week 8						
n	8	8	4	4		
Mean (SD)	10.42 (19.796)	10.42 (19.796)	12.50 (15.957)	-4.17 (20.972)		
Median	0.00	0.00	8.33	0.00		
Min, Max	0.0, 50.0	0.0, 50.0	0.0, 33.3	-33.3, 16.7		

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Modified Randomized Set

Pain

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	8	8	4	4		
Mean (SD)	4.17 (11.785)	4.17 (11.785)	29.17 (8.333)	12.50 (25.000)		
Median	0.00	0.00	33.33	16.67		
Min, Max	0.0, 33.3	0.0, 33.3	16.7, 33.3	-16.7, 33.3		
Week 24						
n	8	8	4	4		
Mean (SD)	0.00 (0.000)	0.00 (0.000)	12.50 (15.957)	-4.17 (20.972)		
Median	0.00	0.00	8.33	0.00		
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 33.3	-33.3, 16.7		

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Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.4.8

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Pain

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	2.78 (6.804)	2.78 (6.804)	16.67 (16.667)	5.56 (25.459)	7.41 (12.108)	3.70 (13.889)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 16.7	0.0, 16.7	0.0, 33.3	-16.7, 33.3	0.0, 33.3	-16.7, 33.3
Week 56						
n	4	4	1	1	5	5
Mean (SD)	25.00 (31.914)	25.00 (31.914)	0.00 (NA)	-33.33 (NA)	20.00 (29.814)	13.33 (38.006)
Median	16.67	16.67	0.00	-33.33	0.00	0.00
Min, Max	0.0, 66.7	0.0, 66.7	0.0, 0.0	-33.3, -33.3	0.0, 66.7	-33.3, 66.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Pain

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	0.00 (NA)			0.00 (NA)	0.00 (NA)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.8

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Dyspnoea

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	45.83 (24.801)		33.33 (27.217)		41.67 (25.126)	
Median	50.00		33.33		33.33	
Min, Max	0.0, 66.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	8	8	4	4		
Mean (SD)	33.33 (30.861)	-12.50 (24.801)	41.67 (16.667)	8.33 (16.667)		
Median	33.33	-16.67	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	33.3, 66.7	0.0, 33.3		

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Modified Randomized Set

Dyspnoea

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	8	8	4	4		
Mean (SD)	25.00 (34.503)	-20.83 (30.538)	41.67 (16.667)	8.33 (16.667)		
Median	16.67	-33.33	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	33.3, 66.7	0.0, 33.3		
Week 8						
n	8	8	4	4		
Mean (SD)	29.17 (33.034)	-16.67 (25.198)	33.33 (0.000)	0.00 (27.217)		
Median	33.33	-33.33	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	33.3, 33.3	-33.3, 33.3		

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Modified Randomized Set

Dyspnoea

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	8	8	4	4		
Mean (SD)	29.17 (21.362)	-16.67 (17.817)	33.33 (0.000)	0.00 (27.217)		
Median	33.33	-16.67	33.33	0.00		
Min, Max	0.0, 66.7	-33.3, 0.0	33.3, 33.3	-33.3, 33.3		
Week 24						
n	8	8	4	4		
Mean (SD)	29.17 (27.817)	-16.67 (25.198)	25.00 (16.667)	-8.33 (16.667)		
Median	33.33	-33.33	33.33	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 33.3	-33.3, 0.0		

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Modified Randomized Set

Dyspnoea

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	16.67 (18.257)	-22.22 (17.213)	11.11 (19.245)	-22.22 (19.245)	14.81 (17.568)	-22.22 (16.667)
Median	16.67	-33.33	0.00	-33.33	0.00	-33.33
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 0.0
Week 56						
n	4	4	1	1	5	5
Mean (SD)	33.33 (27.217)	0.00 (0.000)	33.33 (NA)	33.33 (NA)	33.33 (23.570)	6.67 (14.907)
Median	33.33	0.00	33.33	33.33	33.33	0.00
Min, Max	0.0, 66.7	0.0, 0.0	33.3, 33.3	33.3, 33.3	0.0, 66.7	0.0, 33.3

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Modified Randomized Set

Dyspnoea

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	1	1	0	0	1	1
Mean (SD)	33.33 (NA)	-33.33 (NA)			33.33 (NA)	-33.33 (NA)
Median	33.33	-33.33			33.33	-33.33
Min, Max	33.3, 33.3	-33.3, -33.3			33.3, 33.3	-33.3, -33.3

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Modified Randomized Set

Insomnia

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	12.50 (17.252)		0.00 (0.000)		8.33 (15.076)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 33.3		0.0, 0.0		0.0, 33.3	
Week 2						
n	8	8	4	4		
Mean (SD)	12.50 (17.252)	0.00 (17.817)	16.67 (19.245)	16.67 (19.245)		
Median	0.00	0.00	16.67	16.67		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	0.0, 33.3		

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Modified Randomized Set

Insomnia

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	8	8	4	4		
Mean (SD)	12.50 (17.252)	0.00 (25.198)	8.33 (16.667)	8.33 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	0.0, 33.3		
Week 8						
n	8	8	4	4		
Mean (SD)	8.33 (15.430)	-4.17 (21.362)	16.67 (19.245)	16.67 (19.245)		
Median	0.00	0.00	16.67	16.67		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	0.0, 33.3		

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Modified Randomized Set

Insomnia

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	8	8	4	4		
Mean (SD)	8.33 (15.430)	-4.17 (21.362)	16.67 (19.245)	16.67 (19.245)		
Median	0.00	0.00	16.67	16.67		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	0.0, 33.3		
Week 24						
n	8	8	4	4		
Mean (SD)	12.50 (24.801)	0.00 (35.635)	8.33 (16.667)	8.33 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 33.3	0.0, 33.3		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Source: ADaM.ADSL, ADaM.ADQS

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Insomnia

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	5.56 (13.608)	-5.56 (25.092)	11.11 (19.245)	11.11 (19.245)	7.41 (14.699)	0.00 (23.570)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3	-33.3, 33.3
Week 56						
n	4	4	1	1	5	5
Mean (SD)	16.67 (33.333)	16.67 (33.333)	33.33 (NA)	33.33 (NA)	20.00 (29.814)	20.00 (29.814)
Median	0.00	0.00	33.33	33.33	0.00	0.00
Min, Max	0.0, 66.7	0.0, 66.7	33.3, 33.3	33.3, 33.3	0.0, 66.7	0.0, 66.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Insomnia

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	0.00 (NA)			0.00 (NA)	0.00 (NA)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Appetite Loss

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	12.50 (24.801)		8.33 (16.667)		11.11 (21.711)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 33.3		0.0, 66.7	
Week 2						
n	8	8	4	4		
Mean (SD)	12.50 (24.801)	0.00 (0.000)	16.67 (19.245)	8.33 (16.667)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 66.7	0.0, 0.0	0.0, 33.3	0.0, 33.3		

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Modified Randomized Set

Appetite Loss

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	8	8	4	4		
Mean (SD)	12.50 (24.801)	0.00 (17.817)	8.33 (16.667)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 33.3	0.0, 0.0		
Week 8						
n	8	8	4	4		
Mean (SD)	20.83 (24.801)	8.33 (15.430)	16.67 (19.245)	8.33 (16.667)		
Median	16.67	0.00	16.67	0.00		
Min, Max	0.0, 66.7	0.0, 33.3	0.0, 33.3	0.0, 33.3		

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Modified Randomized Set

Appetite Loss

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	8	8	4	4		
Mean (SD)	8.33 (15.430)	-4.17 (21.362)	25.00 (31.914)	16.67 (19.245)		
Median	0.00	0.00	16.67	16.67		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	0.0, 33.3		
Week 24						
n	8	8	4	4		
Mean (SD)	8.33 (15.430)	-4.17 (33.034)	25.00 (31.914)	16.67 (19.245)		
Median	0.00	0.00	16.67	16.67		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	0.0, 33.3		

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Modified Randomized Set

Appetite Loss

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	5.56 (13.608)	5.56 (13.608)	11.11 (19.245)	0.00 (0.000)	7.41 (14.699)	3.70 (11.111)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 0.0	0.0, 33.3	0.0, 33.3
Week 56						
n	4	4	1	1	5	5
Mean (SD)	16.67 (19.245)	16.67 (19.245)	0.00 (NA)	0.00 (NA)	13.33 (18.257)	13.33 (18.257)
Median	16.67	16.67	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 0.0	0.0, 0.0	0.0, 33.3	0.0, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Appetite Loss

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	0.00 (NA)			0.00 (NA)	0.00 (NA)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Constipation

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	20.83 (35.355)		0.00 (0.000)		13.89 (30.011)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 0.0		0.0, 100.0	
Week 2						
n	8	8	4	4		
Mean (SD)	12.50 (35.355)	-8.33 (15.430)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 0.0	0.0, 0.0	0.0, 0.0		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties. Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem. Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Modified Randomized Set

Constipation

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	8	8	4	4		
Mean (SD)	20.83 (35.355)	0.00 (17.817)	8.33 (16.667)	8.33 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 33.3	0.0, 33.3		
Week 8						
n	8	8	4	4		
Mean (SD)	8.33 (23.570)	-12.50 (17.252)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 0.0	0.0, 0.0		

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Constipation

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	8	8	4	4		
Mean (SD)	16.67 (35.635)	-4.17 (21.362)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 0.0	0.0, 0.0		
Week 24						
n	8	8	4	4		
Mean (SD)	16.67 (35.635)	-4.17 (21.362)	16.67 (19.245)	16.67 (19.245)		
Median	0.00	0.00	16.67	16.67		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 33.3	0.0, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Constipation

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	5.56 (13.608)	-16.67 (27.889)	11.11 (19.245)	11.11 (19.245)	7.41 (14.699)	-7.41 (27.778)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 33.3	0.0, 33.3	0.0, 33.3	-66.7, 33.3
Week 56						
n	4	4	1	1	5	5
Mean (SD)	25.00 (50.000)	0.00 (0.000)	0.00 (NA)	0.00 (NA)	20.00 (44.721)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 100.0	0.0, 0.0

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Modified Randomized Set

Constipation

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	1	1	0	0	1	1
Mean (SD)	100.00 (NA)	0.00 (NA)			100.00 (NA)	0.00 (NA)
Median	100.00	0.00			100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0			100.0, 100.0	0.0, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Diarrhea

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	0.00 (0.000)		0.00 (0.000)		0.00 (0.000)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 0.0		0.0, 0.0		0.0, 0.0	
Week 2						
n	8	8	4	4		
Mean (SD)	0.00 (0.000)	0.00 (0.000)	8.33 (16.667)	8.33 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 33.3	0.0, 33.3		

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Modified Randomized Set

Diarrhea

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	8	8	4	4		
Mean (SD)	8.33 (15.430)	8.33 (15.430)	8.33 (16.667)	8.33 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3		
Week 8						
n	8	8	4	4		
Mean (SD)	4.17 (11.785)	4.17 (11.785)	8.33 (16.667)	8.33 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3		

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Modified Randomized Set

Diarrhea

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	8	8	4	4		
Mean (SD)	4.17 (11.785)	4.17 (11.785)	8.33 (16.667)	8.33 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3		
Week 24						
n	8	8	4	4		
Mean (SD)	8.33 (15.430)	8.33 (15.430)	0.00 (0.000)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 0.0	0.0, 0.0		

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Modified Randomized Set

Diarrhea

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	11.11 (17.213)	11.11 (17.213)	11.11 (19.245)	11.11 (19.245)	11.11 (16.667)	11.11 (16.667)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3
Week 56						
n	4	4	1	1	5	5
Mean (SD)	16.67 (19.245)	16.67 (19.245)	0.00 (NA)	0.00 (NA)	13.33 (18.257)	13.33 (18.257)
Median	16.67	16.67	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 0.0	0.0, 0.0	0.0, 33.3	0.0, 33.3

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Modified Randomized Set

Diarrhea

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	0.00 (NA)			0.00 (NA)	0.00 (NA)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Modified Randomized Set

Financial Difficulties

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	29.17 (45.207)		16.67 (33.333)		25.00 (40.514)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	8	8	4	4		
Mean (SD)	20.83 (30.538)	-8.33 (15.430)	8.33 (16.667)	-8.33 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 33.3	-33.3, 0.0		

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Modified Randomized Set

Financial Difficulties

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	8	8	4	4		
Mean (SD)	20.83 (24.801)	-8.33 (29.547)	8.33 (16.667)	-8.33 (16.667)		
Median	16.67	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 33.3	-33.3, 0.0		
Week 8						
n	8	8	4	4		
Mean (SD)	8.33 (15.430)	-20.83 (43.416)	8.33 (16.667)	-8.33 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	-33.3, 0.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Financial Difficulties

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	8	8	4	4		
Mean (SD)	16.67 (25.198)	-12.50 (30.538)	8.33 (16.667)	-8.33 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 33.3	-33.3, 0.0		
Week 24						
n	8	8	4	4		
Mean (SD)	4.17 (11.785)	-25.00 (38.832)	8.33 (16.667)	-8.33 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-100.0, 0.0	0.0, 33.3	-33.3, 0.0		

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Table GERMANY.2.5.4.8

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Financial Difficulties

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	11.11 (17.213)	-27.78 (32.773)	11.11 (19.245)	-11.11 (19.245)	11.11 (16.667)	-22.22 (28.868)
Median	0.00	-16.67	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-66.7, 0.0
Week 56						
n	4	4	1	1	5	5
Mean (SD)	16.67 (33.333)	-16.67 (19.245)	0.00 (NA)	0.00 (NA)	13.33 (29.814)	-13.33 (18.257)
Median	0.00	-16.67	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 0.0	0.0, 0.0	0.0, 66.7	-33.3, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Financial Difficulties

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	1	1	0	0	1	1
Mean (SD)	0.00 (NA)	0.00 (NA)			0.00 (NA)	0.00 (NA)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.8

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Fatigue

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	49		20		69	
Mean (SD)	43.31 (27.985)		52.22 (27.240)		45.89 (27.870)	
Median	33.33		50.00		44.44	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	47	47	21	19		
Mean (SD)	31.91 (26.275)	-11.82 (22.630)	47.09 (24.064)	-2.34 (23.882)		
Median	33.33	-11.11	44.44	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	11.1, 100.0	-44.4, 44.4		

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Modified Randomized Set

Fatigue

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	49	49	21	20		
Mean (SD)	27.10 (22.840)	-16.21 (24.265)	48.68 (24.715)	-1.67 (25.047)		
Median	22.22	-11.11	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 44.4	11.1, 100.0	-66.7, 44.4		
Week 8						
n	37	37	15	14		
Mean (SD)	27.03 (21.839)	-16.52 (24.931)	40.00 (21.331)	-9.52 (27.857)		
Median	33.33	-22.22	33.33	-5.56		
Min, Max	0.0, 100.0	-66.7, 44.4	0.0, 77.8	-77.8, 44.4		

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Modified Randomized Set

Fatigue

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	44	44	21	20		
Mean (SD)	26.52 (20.080)	-16.67 (20.683)	49.74 (30.352)	-3.89 (34.441)		
Median	33.33	-11.11	44.44	-5.56		
Min, Max	0.0, 66.7	-66.7, 22.2	0.0, 100.0	-77.8, 77.8		
Week 24						
n	41	41	20	19		
Mean (SD)	25.20 (24.091)	-17.62 (26.641)	36.11 (30.984)	-13.45 (32.831)		
Median	22.22	-11.11	33.33	-22.22		
Min, Max	0.0, 100.0	-77.8, 33.3	0.0, 100.0	-55.6, 77.8		

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Modified Randomized Set

Fatigue

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	40	40	18	18	58	58
Mean (SD)	26.67 (23.976)	-15.56 (21.616)	44.44 (33.874)	-3.09 (32.069)	32.18 (28.361)	-11.69 (25.696)
Median	22.22	-11.11	33.33	0.00	33.33	-11.11
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-55.6, 66.7	0.0, 100.0	-66.7, 66.7
Week 56						
n	30	30	16	15	46	45
Mean (SD)	29.63 (21.705)	-12.96 (26.112)	34.72 (14.558)	-12.59 (27.815)	31.40 (19.501)	-12.84 (26.374)
Median	33.33	-11.11	33.33	-11.11	33.33	-11.11
Min, Max	0.0, 100.0	-66.7, 44.4	11.1, 66.7	-66.7, 44.4	0.0, 100.0	-66.7, 44.4

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Modified Randomized Set

Fatigue

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	20	20	12	11	32	31
Mean (SD)	25.56 (20.104)	-20.00 (21.509)	49.07 (22.453)	-1.01 (27.869)	34.38 (23.673)	-13.26 (25.243)
Median	22.22	-11.11	38.89	0.00	33.33	-11.11
Min, Max	0.0, 66.7	-66.7, 22.2	22.2, 100.0	-33.3, 66.7	0.0, 100.0	-66.7, 66.7
Week 88						
n	14	14	8	8	22	22
Mean (SD)	20.63 (21.287)	-20.63 (25.359)	41.67 (22.810)	-13.89 (33.465)	28.28 (23.689)	-18.18 (27.972)
Median	11.11	-22.22	38.89	-16.67	33.33	-22.22
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 77.8	-66.7, 44.4	0.0, 77.8	-66.7, 44.4

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Modified Randomized Set

Fatigue

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	4	4	2	2	6	6
Mean (SD)	13.89 (16.667)	-25.00 (24.637)	27.78 (7.857)	-38.89 (54.997)	18.52 (15.181)	-29.63 (31.946)
Median	11.11	-22.22	27.78	-38.89	22.22	-22.22
Min, Max	0.0, 33.3	-55.6, 0.0	22.2, 33.3	-77.8, 0.0	0.0, 33.3	-77.8, 0.0

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Modified Randomized Set

Nausea and Vomiting

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	49		20		69	
Mean (SD)	3.06 (11.115)		7.50 (16.645)		4.35 (12.989)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	47	47	21	19		
Mean (SD)	4.61 (11.369)	1.42 (14.674)	8.73 (15.473)	0.00 (9.623)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 50.0	-50.0, 50.0	0.0, 50.0	-16.7, 16.7		

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Nausea and Vomiting

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	49	49	21	20		
Mean (SD)	4.76 (10.206)	1.70 (13.283)	3.97 (8.983)	-3.33 (17.605)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-50.0, 33.3	0.0, 33.3	-66.7, 16.7		
Week 8						
n	37	37	15	14		
Mean (SD)	5.41 (15.740)	1.35 (17.293)	7.78 (13.897)	1.19 (22.134)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 83.3	-33.3, 83.3	0.0, 50.0	-66.7, 33.3		

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Modified Randomized Set

Nausea and Vomiting

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	44	44	21	20		
Mean (SD)	3.79 (9.417)	0.76 (11.341)	9.52 (20.798)	0.00 (20.943)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-50.0, 33.3	0.0, 83.3	-66.7, 50.0		
Week 24						
n	41	41	20	19		
Mean (SD)	3.66 (12.083)	0.41 (15.133)	4.17 (7.404)	0.00 (13.608)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-50.0, 66.7	0.0, 16.7	-33.3, 16.7		

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Nausea and Vomiting

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	40	40	18	18	58	58
Mean (SD)	5.42 (9.539)	3.75 (9.613)	5.56 (12.783)	1.85 (15.004)	5.46 (10.535)	3.16 (11.452)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-16.7, 33.3	0.0, 50.0	-16.7, 50.0	0.0, 50.0	-16.7, 50.0
Week 56						
n	30	30	16	15	46	45
Mean (SD)	0.56 (3.043)	-1.67 (8.011)	10.42 (25.000)	6.67 (24.234)	3.99 (15.390)	1.11 (15.651)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	-33.3, 16.7	0.0, 100.0	-33.3, 83.3	0.0, 100.0	-33.3, 83.3

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Modified Randomized Set

Nausea and Vomiting

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	20	20	12	11	32	31
Mean (SD)	0.83 (3.727)	-0.83 (6.567)	6.94 (11.143)	3.03 (17.979)	3.13 (7.849)	0.54 (11.772)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	-16.7, 16.7	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3
Week 88						
n	14	14	8	8	22	22
Mean (SD)	1.19 (4.454)	0.00 (0.000)	8.33 (8.909)	2.08 (16.517)	3.79 (7.149)	0.76 (9.591)
Median	0.00	0.00	8.33	0.00	0.00	0.00
Min, Max	0.0, 16.7	0.0, 0.0	0.0, 16.7	-33.3, 16.7	0.0, 16.7	-33.3, 16.7

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Modified Randomized Set

Nausea and Vomiting

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	4	4	2	2	6	6
Mean (SD)	0.00 (0.000)	0.00 (0.000)	50.00 (23.570)	33.33 (0.000)	16.67 (27.889)	11.11 (17.213)
Median	0.00	0.00	50.00	33.33	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	33.3, 66.7	33.3, 33.3	0.0, 66.7	0.0, 33.3

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Modified Randomized Set

Pain

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	49		20		69	
Mean (SD)	17.01 (26.460)		21.67 (27.091)		18.36 (26.529)	
Median	0.00		16.67		0.00	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	47	47	20	18		
Mean (SD)	14.18 (24.813)	-3.55 (18.694)	25.00 (23.258)	0.93 (20.189)		
Median	0.00	0.00	25.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 66.7	-33.3, 33.3		

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Modified Randomized Set

Pain

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	49	49	21	20		
Mean (SD)	13.95 (22.399)	-3.06 (15.838)	20.63 (30.233)	0.00 (24.780)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 83.3		
Week 8						
n	36	36	14	13		
Mean (SD)	12.96 (25.854)	-0.46 (28.308)	9.52 (12.599)	-10.26 (17.398)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 33.3	-33.3, 16.7		

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Pain

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	44	44	20	19		
Mean (SD)	12.50 (23.877)	-4.92 (21.440)	23.33 (32.173)	0.88 (31.659)		
Median	0.00	0.00	8.33	0.00		
Min, Max	0.0, 100.0	-83.3, 33.3	0.0, 100.0	-33.3, 83.3		
Week 24						
n	41	41	20	19		
Mean (SD)	13.82 (25.523)	-4.07 (25.490)	14.17 (22.475)	-6.14 (22.368)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-83.3, 66.7	0.0, 83.3	-66.7, 33.3		

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Pain

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	40	40	18	18	58	58
Mean (SD)	14.58 (24.803)	-2.92 (21.313)	26.85 (36.218)	8.33 (34.419)	18.39 (29.068)	0.57 (26.300)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 83.3	-83.3, 33.3	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-83.3, 100.0
Week 56						
n	30	30	16	15	46	45
Mean (SD)	16.11 (26.437)	-5.56 (22.883)	27.08 (22.669)	8.89 (25.871)	19.93 (25.488)	-0.74 (24.607)
Median	0.00	0.00	25.00	0.00	8.33	0.00
Min, Max	0.0, 100.0	-83.3, 33.3	0.0, 66.7	-33.3, 66.7	0.0, 100.0	-83.3, 66.7

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Table GERMANY.2.5.4.8

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Pain

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	19	19	12	11	31	30
Mean (SD)	8.77 (17.004)	-9.65 (20.273)	22.22 (30.429)	9.09 (33.635)	13.98 (23.608)	-2.78 (27.010)
Median	0.00	0.00	8.33	0.00	0.00	0.00
Min, Max	0.0, 66.7	-50.0, 16.7	0.0, 100.0	-33.3, 83.3	0.0, 100.0	-50.0, 83.3
Week 88						
n	14	14	8	8	22	22
Mean (SD)	9.52 (19.298)	-8.33 (20.412)	25.00 (29.547)	16.67 (32.121)	15.15 (24.075)	0.76 (27.448)
Median	0.00	0.00	16.67	8.33	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 16.7	0.0, 83.3	-33.3, 66.7	0.0, 83.3	-66.7, 66.7

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.4.8

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Pain

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	4	4	2	2	6	6
Mean (SD)	8.33 (16.667)	-16.67 (33.333)	16.67 (0.000)	8.33 (11.785)	11.11 (13.608)	-8.33 (29.345)
Median	0.00	0.00	16.67	8.33	8.33	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	16.7, 16.7	0.0, 16.7	0.0, 33.3	-66.7, 16.7

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Modified Randomized Set

Dyspnoea

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	49		20		69	
Mean (SD)	35.37 (29.193)		30.00 (30.397)		33.82 (29.424)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	48	48	21	19		
Mean (SD)	20.83 (25.381)	-15.28 (22.762)	31.75 (28.822)	3.51 (29.175)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Modified Randomized Set

Dyspnoea

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	49	49	21	20		
Mean (SD)	12.93 (20.203)	-22.45 (24.896)	31.75 (35.709)	3.33 (35.705)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-66.7, 66.7		
Week 8						
n	37	37	15	14		
Mean (SD)	16.22 (20.217)	-18.02 (24.336)	26.67 (31.371)	-7.14 (37.390)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 66.7	-100.0, 33.3	0.0, 100.0	-100.0, 66.7		

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Modified Randomized Set

Dyspnoea

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	44	44	21	20		
Mean (SD)	14.39 (20.832)	-20.45 (26.134)	28.57 (26.427)	-1.67 (42.543)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-100.0, 66.7		
Week 24						
n	41	41	20	19		
Mean (SD)	13.82 (21.050)	-19.51 (27.864)	13.33 (16.754)	-12.28 (27.690)		
Median	0.00	-33.33	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 33.3	-66.7, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Dyspnoea

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	40	40	18	18	58	58
Mean (SD)	17.50 (25.021)	-19.17 (24.907)	16.67 (17.150)	-11.11 (22.866)	17.24 (22.721)	-16.67 (24.383)
Median	0.00	-33.33	16.67	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 100.0	-66.7, 33.3
Week 56						
n	30	30	16	15	46	45
Mean (SD)	21.11 (28.343)	-15.56 (27.310)	10.42 (15.957)	-11.11 (24.125)	17.39 (25.082)	-14.07 (26.101)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 100.0	-66.7, 33.3

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Table GERMANY.2.5.4.8

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Dyspnoea

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	20	20	12	11	32	31
Mean (SD)	13.33 (22.685)	-26.67 (20.520)	19.44 (26.432)	-9.09 (33.635)	15.63 (23.924)	-20.43 (26.774)
Median	0.00	-33.33	0.00	0.00	0.00	-33.33
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-66.7, 33.3
Week 88						
n	14	14	8	8	22	22
Mean (SD)	7.14 (19.298)	-26.19 (23.310)	8.33 (15.430)	-16.67 (30.861)	7.58 (17.614)	-22.73 (25.996)
Median	0.00	-33.33	0.00	-16.67	0.00	-33.33
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-66.7, 33.3

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Modified Randomized Set

Dyspnoea

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	4	4	2	2	6	6
Mean (SD)	0.00 (0.000)	-25.00 (31.914)	16.67 (23.570)	0.00 (0.000)	5.56 (13.608)	-16.67 (27.889)
Median	0.00	-16.67	16.67	0.00	0.00	0.00
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 33.3	0.0, 0.0	0.0, 33.3	-66.7, 0.0

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Modified Randomized Set

Insomnia

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	49		20		69	
Mean (SD)	28.57 (28.868)		35.00 (31.484)		30.43 (29.561)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	47	47	21	19		
Mean (SD)	23.40 (31.786)	-5.67 (20.047)	31.75 (34.118)	-3.51 (33.138)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 66.7		

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Modified Randomized Set

Insomnia

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	49	49	21	20		
Mean (SD)	22.45 (32.905)	-6.12 (24.223)	28.57 (32.121)	-6.67 (38.389)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-100.0, 66.7		
Week 8						
n	37	37	15	14		
Mean (SD)	14.41 (20.093)	-14.41 (25.508)	15.56 (27.794)	-14.29 (31.254)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-100.0, 33.3		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Table GERMANY.2.5.4.8

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Insomnia

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	44	44	21	20		
Mean (SD)	18.18 (25.370)	-10.61 (20.042)	33.33 (34.960)	-1.67 (36.635)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 66.7		
Week 24						
n	41	41	20	19		
Mean (SD)	15.45 (25.925)	-12.20 (26.621)	23.33 (24.423)	-12.28 (25.363)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 66.7	-66.7, 33.3		

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Modified Randomized Set

Insomnia

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	40	40	18	18	58	58
Mean (SD)	22.50 (28.632)	-6.67 (24.108)	27.78 (34.773)	-7.41 (42.095)	24.14 (30.457)	-6.90 (30.435)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-100.0, 66.7	0.0, 100.0	-100.0, 66.7
Week 56						
n	30	30	16	15	46	45
Mean (SD)	22.22 (26.743)	-6.67 (23.813)	37.50 (34.157)	0.00 (33.333)	27.54 (30.065)	-4.44 (27.155)
Median	33.33	0.00	33.33	0.00	33.33	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7

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Modified Randomized Set

Insomnia

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	20	20	12	11	32	31
Mean (SD)	13.33 (19.941)	-13.33 (19.941)	38.89 (34.329)	-3.03 (48.200)	22.92 (28.634)	-9.68 (32.425)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 100.0	-100.0, 66.7	0.0, 100.0	-100.0, 66.7
Week 88						
n	14	14	8	8	22	22
Mean (SD)	9.52 (20.375)	-11.90 (16.575)	41.67 (42.725)	0.00 (47.140)	21.21 (33.405)	-7.58 (30.744)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7

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Modified Randomized Set

Insomnia

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	4	4	2	2	6	6
Mean (SD)	8.33 (16.667)	-16.67 (33.333)	50.00 (70.711)	-16.67 (117.851)	22.22 (40.369)	-16.67 (58.689)
Median	0.00	0.00	50.00	-16.67	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 100.0	-100.0, 66.7	0.0, 100.0	-100.0, 66.7

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Modified Randomized Set

Appetite Loss

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	49		20		69	
Mean (SD)	8.16 (14.483)		20.00 (29.419)		11.59 (20.473)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 33.3		0.0, 100.0		0.0, 100.0	
Week 2						
n	48	48	21	19		
Mean (SD)	9.72 (18.138)	1.39 (20.581)	20.63 (24.667)	0.00 (15.713)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-33.3, 33.3		

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Modified Randomized Set

Appetite Loss

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	49	49	21	20		
Mean (SD)	4.76 (11.785)	-3.40 (14.019)	14.29 (29.005)	-5.00 (29.170)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-66.7, 66.7		
Week 8						
n	37	37	15	14		
Mean (SD)	11.71 (17.944)	0.90 (21.497)	11.11 (20.574)	-7.14 (29.753)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-100.0, 33.3		

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Appetite Loss

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	44	44	21	20		
Mean (SD)	6.82 (19.792)	-0.76 (25.405)	15.87 (29.096)	-3.33 (23.939)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-66.7, 33.3		
Week 24						
n	41	41	20	19		
Mean (SD)	11.38 (24.278)	4.07 (28.082)	15.00 (22.878)	0.00 (11.111)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 66.7	-33.3, 33.3		

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Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	40	40	18	18	58	58
Mean (SD)	6.67 (13.503)	-1.67 (16.794)	20.37 (28.328)	7.41 (29.273)	10.92 (20.124)	1.15 (21.598)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0
Week 56						
n	30	30	16	15	46	45
Mean (SD)	6.67 (13.561)	0.00 (19.574)	12.50 (26.874)	-2.22 (26.627)	8.70 (19.161)	-0.74 (21.891)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7

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Modified Randomized Set

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Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	20	20	12	11	32	31
Mean (SD)	3.33 (10.260)	-5.00 (12.212)	19.44 (22.285)	3.03 (23.355)	9.38 (17.422)	-2.15 (17.074)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-33.3, 66.7
Week 88						
n	14	14	8	8	22	22
Mean (SD)	7.14 (14.194)	0.00 (13.074)	16.67 (17.817)	0.00 (30.861)	10.61 (15.891)	0.00 (20.574)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3

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Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.4.8

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Appetite Loss

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	4	4	2	2	6	6
Mean (SD)	0.00 (0.000)	0.00 (0.000)	66.67 (0.000)	16.67 (23.570)	22.22 (34.427)	5.56 (13.608)
Median	0.00	0.00	66.67	16.67	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	0.0, 33.3	0.0, 66.7	0.0, 33.3

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Table GERMANY.2.5.4.8

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Modified Randomized Set

Constipation

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	49		20		69	
Mean (SD)	12.24 (21.185)		13.33 (19.941)		12.56 (20.692)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	47	47	21	19		
Mean (SD)	9.22 (20.503)	-3.55 (19.944)	20.63 (32.449)	7.02 (28.499)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

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Modified Randomized Set

Constipation

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	49	49	21	20		
Mean (SD)	8.16 (19.873)	-4.08 (22.187)	20.63 (30.689)	8.33 (26.213)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 66.7	0.0, 100.0	-33.3, 66.7		
Week 8						
n	37	37	15	14		
Mean (SD)	5.41 (12.456)	-5.41 (21.536)	20.00 (32.854)	9.52 (15.627)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 100.0	0.0, 33.3		

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Modified Randomized Set

Constipation

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	44	44	21	20		
Mean (SD)	8.33 (21.716)	-3.79 (20.606)	20.63 (34.118)	8.33 (23.878)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-33.3, 66.7		
Week 24						
n	41	41	20	19		
Mean (SD)	4.88 (14.065)	-6.50 (20.027)	16.67 (27.572)	3.51 (21.928)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

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Modified Randomized Set

Constipation

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	40	40	18	18	58	58
Mean (SD)	6.67 (15.470)	-7.50 (20.655)	16.67 (23.570)	3.70 (22.547)	9.77 (18.741)	-4.02 (21.699)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-66.7, 66.7
Week 56						
n	30	30	16	15	46	45
Mean (SD)	7.78 (16.800)	-8.89 (21.323)	12.50 (20.638)	0.00 (12.599)	9.42 (18.139)	-5.93 (19.187)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-66.7, 33.3

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Modified Randomized Set

Constipation

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	20	20	12	11	32	31
Mean (SD)	8.33 (14.809)	-8.33 (21.289)	11.11 (16.412)	3.03 (17.979)	9.38 (15.227)	-4.30 (20.623)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3
Week 88						
n	14	14	8	8	22	22
Mean (SD)	14.29 (17.118)	-4.76 (22.100)	25.00 (46.291)	16.67 (43.644)	18.18 (30.389)	3.03 (32.382)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0

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Modified Randomized Set

Constipation

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	4	4	2	2	6	6
Mean (SD)	16.67 (19.245)	-8.33 (16.667)	33.33 (47.140)	0.00 (47.140)	22.22 (27.217)	-5.56 (25.092)
Median	16.67	0.00	33.33	0.00	16.67	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3

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Modified Randomized Set

Diarrhea

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	49		20		69	
Mean (SD)	6.12 (17.577)		15.00 (22.878)		8.70 (19.514)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	48	48	21	19		
Mean (SD)	6.94 (19.398)	0.69 (18.818)	4.76 (15.936)	-10.53 (22.368)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 100.0	0.0, 66.7	-66.7, 33.3		

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Modified Randomized Set

Diarrhea

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	49	49	21	20		
Mean (SD)	4.76 (13.608)	-1.36 (20.366)	3.17 (10.026)	-11.67 (19.571)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-100.0, 66.7	0.0, 33.3	-66.7, 0.0		
Week 8						
n	37	37	15	14		
Mean (SD)	8.11 (16.491)	1.80 (20.707)	6.67 (18.687)	-11.90 (16.575)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 66.7	0.0, 66.7	-33.3, 0.0		

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Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	44	44	21	20		
Mean (SD)	6.06 (13.005)	-0.76 (16.842)	3.17 (14.548)	-11.67 (29.170)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-66.7, 66.7		
Week 24						
n	41	41	20	19		
Mean (SD)	4.88 (11.928)	-0.81 (15.790)	5.00 (12.212)	-8.77 (18.732)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 0.0		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties. Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem. Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan. D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point. Baseline is defined as the last non-missing value prior to the first dose of study drug. Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.4.8

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Diarrhea

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	40	40	18	18	58	58
Mean (SD)	4.17 (11.164)	-3.33 (21.082)	7.41 (14.260)	-7.41 (24.403)	5.17 (12.174)	-4.60 (22.030)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-100.0, 33.3
Week 56						
n	30	30	16	15	46	45
Mean (SD)	5.56 (12.635)	0.00 (21.442)	8.33 (14.907)	-11.11 (24.125)	6.52 (13.370)	-3.70 (22.722)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-100.0, 33.3

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Diarrhea

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	19	19	12	11	31	30
Mean (SD)	0.00 (0.000)	-1.75 (7.647)	25.00 (32.177)	6.06 (35.957)	9.68 (23.084)	1.11 (22.289)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0
Week 88						
n	14	14	8	8	22	22
Mean (SD)	2.38 (8.909)	2.38 (8.909)	8.33 (15.430)	-8.33 (29.547)	4.55 (11.708)	-1.52 (19.182)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Diarrhea

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	4	4	2	2	6	6
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0

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Modified Randomized Set

Financial Difficulties

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	49		20		69	
Mean (SD)	19.05 (28.054)		25.00 (32.218)		20.77 (29.206)	
Median	0.00		16.67		0.00	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	49	49	21	19		
Mean (SD)	17.69 (29.738)	-1.36 (15.152)	26.98 (35.931)	5.26 (29.945)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 100.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Financial Difficulties

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	49	49	21	20		
Mean (SD)	17.01 (27.320)	-2.04 (18.519)	20.63 (35.709)	-3.33 (32.264)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 100.0		
Week 8						
n	37	37	15	14		
Mean (SD)	13.51 (22.851)	-3.60 (13.109)	15.56 (24.774)	2.38 (20.524)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 66.7	-33.3, 33.3		

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Modified Randomized Set

Financial Difficulties

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	44	44	21	20		
Mean (SD)	18.18 (27.331)	-0.76 (13.427)	25.40 (37.866)	1.67 (35.002)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 100.0		
Week 24						
n	41	41	20	19		
Mean (SD)	20.33 (30.621)	1.63 (19.651)	25.00 (37.268)	0.00 (31.427)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-66.7, 100.0		

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Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	40	40	18	18	58	58
Mean (SD)	18.33 (28.193)	-1.67 (19.900)	22.22 (34.300)	-5.56 (38.348)	19.54 (29.967)	-2.87 (26.699)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-100.0, 100.0	0.0, 100.0	-100.0, 100.0
Week 56						
n	30	30	16	15	46	45
Mean (SD)	12.22 (22.289)	-7.78 (18.944)	27.08 (34.894)	-2.22 (40.760)	17.39 (27.879)	-5.93 (27.788)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 100.0	0.0, 100.0	-66.7, 100.0

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Modified Randomized Set

Financial Difficulties

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	19	19	12	11	31	30
Mean (SD)	8.77 (15.080)	-10.53 (24.976)	11.11 (21.711)	-15.15 (22.918)	9.68 (17.625)	-12.22 (23.947)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-66.7, 0.0	0.0, 66.7	-66.7, 33.3
Week 88						
n	14	14	8	8	22	22
Mean (SD)	9.52 (15.627)	-9.52 (20.375)	8.33 (15.430)	-25.00 (29.547)	9.09 (15.195)	-15.15 (24.618)
Median	0.00	0.00	0.00	-16.67	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0

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Modified Randomized Set

Financial Difficulties

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	4	4	2	2	6	6
Mean (SD)	0.00 (0.000)	-16.67 (33.333)	16.67 (23.570)	-66.67 (0.000)	5.56 (13.608)	-33.33 (36.515)
Median	0.00	0.00	16.67	-66.67	0.00	-33.33
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 33.3	-66.7, -66.7	0.0, 33.3	-66.7, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1
and Period 2 and Long Term Extension by Race
Modified Randomized Set

Fatigue

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	37.88 (25.118)		45.56 (32.054)		40.28 (27.180)	
Median	33.33		38.89		33.33	
Min, Max	0.0, 88.9		0.0, 100.0		0.0, 100.0	
Week 2						
n	22	22	10	10		
Mean (SD)	29.29 (19.860)	-8.59 (18.122)	34.44 (13.302)	-11.11 (22.831)		
Median	33.33	0.00	33.33	-5.56		
Min, Max	0.0, 66.7	-55.6, 22.2	11.1, 55.6	-44.4, 11.1		

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Modified Randomized Set

Fatigue

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	10		
Mean (SD)	27.78 (21.068)	-10.10 (22.198)	41.11 (21.628)	-4.44 (26.810)		
Median	27.78	0.00	33.33	5.56		
Min, Max	0.0, 77.8	-66.7, 22.2	11.1, 88.9	-66.7, 22.2		
Week 8						
n	18	18	10	10		
Mean (SD)	29.63 (20.166)	-6.79 (18.724)	30.00 (16.605)	-15.56 (26.294)		
Median	33.33	0.00	27.78	-11.11		
Min, Max	0.0, 77.8	-44.4, 22.2	0.0, 55.6	-77.8, 11.1		

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Modified Randomized Set

Fatigue

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	10		
Mean (SD)	22.78 (18.549)	-13.89 (24.417)	33.33 (14.815)	-12.22 (28.903)		
Median	22.22	-5.56	33.33	-5.56		
Min, Max	0.0, 55.6	-66.7, 22.2	11.1, 66.7	-77.8, 22.2		
Week 24						
n	20	20	9	9		
Mean (SD)	21.11 (17.624)	-15.56 (24.290)	30.86 (27.090)	-8.64 (20.621)		
Median	22.22	-11.11	22.22	0.00		
Min, Max	0.0, 66.7	-77.8, 33.3	0.0, 88.9	-44.4, 22.2		

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Modified Randomized Set

Fatigue

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	7	7	27	27
Mean (SD)	18.89 (14.465)	-17.22 (23.494)	28.57 (23.880)	-6.35 (16.798)	21.40 (17.412)	-14.40 (22.183)
Median	22.22	-11.11	33.33	0.00	22.22	-11.11
Min, Max	0.0, 44.4	-77.8, 11.1	0.0, 66.7	-33.3, 11.1	0.0, 66.7	-77.8, 11.1
Week 56						
n	16	16	5	5	21	21
Mean (SD)	28.47 (20.675)	-9.03 (26.362)	31.11 (16.480)	-4.44 (23.040)	29.10 (19.397)	-7.94 (25.128)
Median	33.33	0.00	33.33	-11.11	33.33	0.00
Min, Max	0.0, 66.7	-66.7, 33.3	11.1, 55.6	-22.2, 33.3	0.0, 66.7	-66.7, 33.3

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Modified Randomized Set

Fatigue

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	3	3	12	12
Mean (SD)	19.75 (13.354)	-23.46 (20.370)	48.15 (16.973)	-3.70 (6.415)	26.85 (18.628)	-18.52 (19.725)
Median	22.22	-11.11	44.44	0.00	27.78	-11.11
Min, Max	0.0, 33.3	-66.7, 0.0	33.3, 66.7	-11.1, 0.0	0.0, 66.7	-66.7, 0.0
Week 88						
n	7	7	2	2	9	9
Mean (SD)	25.40 (21.956)	-19.05 (23.757)	27.78 (39.284)	-22.22 (15.713)	25.93 (23.570)	-19.75 (21.357)
Median	22.22	-22.22	27.78	-22.22	22.22	-22.22
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 55.6	-33.3, -11.1	0.0, 66.7	-66.7, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Fatigue

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	0	0	2	2
Mean (SD)	11.11 (15.713)	-22.22 (15.713)			11.11 (15.713)	-22.22 (15.713)
Median	11.11	-22.22			11.11	-22.22
Min, Max	0.0, 22.2	-33.3, -11.1			0.0, 22.2	-33.3, -11.1

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Modified Randomized Set

Nausea and Vomiting

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	0.76 (3.553)		10.00 (21.082)		3.65 (12.511)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 16.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	22	22	10	10		
Mean (SD)	0.76 (3.553)	0.00 (0.000)	5.00 (15.811)	-5.00 (8.051)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 16.7	0.0, 0.0	0.0, 50.0	-16.7, 0.0		

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Nausea and Vomiting

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	10		
Mean (SD)	0.76 (3.553)	0.00 (0.000)	5.00 (11.249)	-5.00 (22.292)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 16.7	0.0, 0.0	0.0, 33.3	-66.7, 16.7		
Week 8						
n	18	18	10	10		
Mean (SD)	1.85 (5.390)	0.93 (3.928)	6.67 (16.102)	-3.33 (24.595)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 16.7	0.0, 16.7	0.0, 50.0	-66.7, 33.3		

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Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	10		
Mean (SD)	1.67 (7.454)	1.67 (7.454)	1.67 (5.270)	-8.33 (21.155)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 16.7	-66.7, 0.0		
Week 24						
n	20	20	9	9		
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	-3.70 (7.349)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	-16.7, 0.0		

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Nausea and Vomiting

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	7	7	27	27
Mean (SD)	0.83 (3.727)	0.00 (0.000)	0.00 (0.000)	-2.38 (6.299)	0.62 (3.208)	-0.62 (3.208)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	0.0, 0.0	0.0, 0.0	-16.7, 0.0	0.0, 16.7	-16.7, 0.0
Week 56						
n	16	16	5	5	21	21
Mean (SD)	0.00 (0.000)	-1.04 (4.167)	3.33 (7.454)	0.00 (0.000)	0.79 (3.637)	-0.79 (3.637)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	-16.7, 0.0	0.0, 16.7	0.0, 0.0	0.0, 16.7	-16.7, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Nausea and Vomiting

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	3	3	12	12
Mean (SD)	0.00 (0.000)	-1.85 (5.556)	0.00 (0.000)	-5.56 (9.623)	0.00 (0.000)	-2.78 (6.487)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	-16.7, 0.0	0.0, 0.0	-16.7, 0.0	0.0, 0.0	-16.7, 0.0
Week 88						
n	7	7	2	2	9	9
Mean (SD)	2.38 (6.299)	0.00 (0.000)	8.33 (11.785)	0.00 (0.000)	3.70 (7.349)	0.00 (0.000)
Median	0.00	0.00	8.33	0.00	0.00	0.00
Min, Max	0.0, 16.7	0.0, 0.0	0.0, 16.7	0.0, 0.0	0.0, 16.7	0.0, 0.0

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Modified Randomized Set

Nausea and Vomiting

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	0	0	2	2
Mean (SD)	0.00 (0.000)	0.00 (0.000)			0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.9
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Modified Randomized Set

Pain

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	10.61 (20.922)		20.00 (23.307)		13.54 (21.767)	
Median	0.00		16.67		0.00	
Min, Max	0.0, 83.3		0.0, 66.7		0.0, 83.3	
Week 2						
n	22	22	10	10		
Mean (SD)	9.85 (18.300)	-0.76 (13.090)	20.00 (20.488)	0.00 (15.713)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 16.7		

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and Period 2 and Long Term Extension by Race
Modified Randomized Set

Pain

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	10		
Mean (SD)	9.09 (16.042)	-1.52 (20.515)	15.00 (22.839)	-5.00 (15.811)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 50.0	-66.7, 50.0	0.0, 66.7	-33.3, 16.7		
Week 8						
n	18	18	10	10		
Mean (SD)	7.41 (14.260)	-0.93 (20.189)	10.00 (14.055)	-10.00 (21.082)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 50.0	-33.3, 50.0	0.0, 33.3	-33.3, 16.7		

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Modified Randomized Set

Pain

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	10		
Mean (SD)	3.33 (8.719)	-5.00 (22.361)	20.00 (17.213)	0.00 (22.222)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 33.3	-83.3, 33.3	0.0, 50.0	-33.3, 33.3		
Week 24						
n	20	20	9	9		
Mean (SD)	3.33 (8.719)	-5.00 (22.361)	16.67 (27.639)	-1.85 (19.444)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-83.3, 33.3	0.0, 83.3	-33.3, 16.7		

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Pain

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	7	7	27	27
Mean (SD)	5.83 (11.180)	-5.83 (23.116)	7.14 (13.113)	-2.38 (20.250)	6.17 (11.462)	-4.94 (22.079)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-83.3, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-83.3, 33.3
Week 56						
n	16	16	5	5	21	21
Mean (SD)	13.54 (19.454)	-1.04 (30.713)	16.67 (23.570)	3.33 (29.814)	14.29 (19.920)	0.00 (29.814)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-83.3, 66.7	0.0, 50.0	-33.3, 50.0	0.0, 66.7	-83.3, 66.7

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Pain

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	3	3	12	12
Mean (SD)	9.26 (12.108)	-7.41 (22.222)	0.00 (0.000)	-11.11 (19.245)	6.94 (11.143)	-8.33 (20.719)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-50.0, 16.7	0.0, 0.0	-33.3, 0.0	0.0, 33.3	-50.0, 16.7
Week 88						
n	7	7	2	2	9	9
Mean (SD)	9.52 (13.113)	-11.90 (26.726)	0.00 (0.000)	-16.67 (23.570)	7.41 (12.108)	-12.96 (24.689)
Median	0.00	0.00	0.00	-16.67	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 16.7	0.0, 0.0	-33.3, 0.0	0.0, 33.3	-66.7, 16.7

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Modified Randomized Set

Pain

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	0	0	2	2
Mean (SD)	0.00 (0.000)	0.00 (0.000)			0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Modified Randomized Set

Dyspnoea

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	37.88 (23.672)		33.33 (35.136)		36.46 (27.251)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 66.7		0.0, 100.0		0.0, 100.0	
Week 2						
n	22	22	10	10		
Mean (SD)	21.21 (26.318)	-16.67 (22.420)	30.00 (24.595)	-3.33 (24.595)		
Median	16.67	-16.67	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	-33.3, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1
and Period 2 and Long Term Extension by Race
Modified Randomized Set

Dyspnoea

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	10		
Mean (SD)	15.15 (24.618)	-22.73 (23.874)	33.33 (22.222)	0.00 (35.136)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	-66.7, 66.7		
Week 8						
n	18	18	10	10		
Mean (SD)	18.52 (26.127)	-20.37 (20.256)	16.67 (17.568)	-16.67 (36.004)		
Median	0.00	-33.33	16.67	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 33.3	-100.0, 33.3		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1
and Period 2 and Long Term Extension by Race
Modified Randomized Set

Dyspnoea

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	10		
Mean (SD)	13.33 (19.941)	-21.67 (19.571)	20.00 (23.307)	-13.33 (47.661)		
Median	0.00	-33.33	16.67	0.00		
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 66.7	-100.0, 66.7		
Week 24						
n	20	20	9	9		
Mean (SD)	15.00 (22.878)	-20.00 (22.685)	14.81 (17.568)	-11.11 (28.868)		
Median	0.00	-33.33	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 33.3	-66.7, 33.3		

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Dyspnoea

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	7	7	27	27
Mean (SD)	8.33 (14.809)	-26.67 (20.520)	9.52 (16.265)	-19.05 (26.227)	8.64 (14.886)	-24.69 (21.863)
Median	0.00	-33.33	0.00	0.00	0.00	-33.33
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0
Week 56						
n	16	16	5	5	21	21
Mean (SD)	14.58 (20.972)	-18.75 (24.248)	13.33 (18.257)	-6.67 (27.889)	14.29 (19.920)	-15.87 (24.987)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-66.7, 33.3

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Dyspnoea

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	3	3	12	12
Mean (SD)	7.41 (14.699)	-37.04 (20.031)	0.00 (0.000)	-33.33 (33.333)	5.56 (12.975)	-36.11 (22.285)
Median	0.00	-33.33	0.00	-33.33	0.00	-33.33
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 0.0	-66.7, 0.0	0.0, 33.3	-66.7, 0.0
Week 88						
n	7	7	2	2	9	9
Mean (SD)	14.29 (26.227)	-28.57 (23.002)	0.00 (0.000)	-33.33 (47.140)	11.11 (23.570)	-29.63 (26.058)
Median	0.00	-33.33	0.00	-33.33	0.00	-33.33
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 0.0	-66.7, 0.0	0.0, 66.7	-66.7, 0.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Dyspnoea

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	0	0	2	2
Mean (SD)	0.00 (0.000)	-16.67 (23.570)			0.00 (0.000)	-16.67 (23.570)
Median	0.00	-16.67			0.00	-16.67
Min, Max	0.0, 0.0	-33.3, 0.0			0.0, 0.0	-33.3, 0.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Insomnia

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	15.15 (19.860)		23.33 (27.442)		17.71 (22.376)	
Median	0.00		16.67		0.00	
Min, Max	0.0, 66.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	22	22	10	10		
Mean (SD)	15.15 (19.860)	0.00 (14.548)	26.67 (30.631)	3.33 (24.595)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-33.3, 33.3		

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and Period 2 and Long Term Extension by Race
Modified Randomized Set

Insomnia

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	10		
Mean (SD)	10.61 (18.930)	-4.55 (18.674)	20.00 (32.203)	-3.33 (24.595)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-33.3, 33.3		
Week 8						
n	18	18	10	10		
Mean (SD)	5.56 (12.783)	-11.11 (19.803)	23.33 (31.623)	0.00 (27.217)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 33.3		

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Insomnia

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	10		
Mean (SD)	6.67 (13.680)	-6.67 (17.438)	26.67 (21.082)	3.33 (24.595)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-33.3, 33.3		
Week 24						
n	20	20	9	9		
Mean (SD)	10.00 (19.041)	-3.33 (23.939)	18.52 (24.216)	-3.70 (26.058)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-66.7, 33.3		

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Insomnia

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	7	7	27	27
Mean (SD)	8.33 (14.809)	-6.67 (23.195)	19.05 (26.227)	-4.76 (29.991)	11.11 (18.490)	-6.17 (24.525)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-66.7, 33.3
Week 56						
n	16	16	5	5	21	21
Mean (SD)	16.67 (21.082)	4.17 (23.960)	40.00 (36.515)	6.67 (27.889)	22.22 (26.527)	4.76 (24.234)
Median	0.00	0.00	33.33	0.00	33.33	0.00
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 66.7

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Modified Randomized Set

Insomnia

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	3	3	12	12
Mean (SD)	11.11 (16.667)	-3.70 (11.111)	44.44 (50.918)	-11.11 (50.918)	19.44 (30.011)	-5.56 (23.925)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 33.3
Week 88						
n	7	7	2	2	9	9
Mean (SD)	14.29 (26.227)	-4.76 (12.599)	50.00 (70.711)	-16.67 (70.711)	22.22 (37.268)	-7.41 (27.778)
Median	0.00	0.00	50.00	-16.67	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 33.3

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Insomnia

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	0	0	2	2
Mean (SD)	16.67 (23.570)	0.00 (0.000)			16.67 (23.570)	0.00 (0.000)
Median	16.67	0.00			16.67	0.00
Min, Max	0.0, 33.3	0.0, 0.0			0.0, 33.3	0.0, 0.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Appetite Loss

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	10.61 (18.930)		26.67 (34.427)		15.63 (25.378)	
Median	0.00		16.67		0.00	
Min, Max	0.0, 66.7		0.0, 100.0		0.0, 100.0	
Week 2						
n	22	22	10	10		
Mean (SD)	7.58 (17.614)	-3.03 (9.808)	23.33 (22.498)	-3.33 (18.922)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 66.7	-33.3, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1
and Period 2 and Long Term Extension by Race
Modified Randomized Set

Appetite Loss

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	10		
Mean (SD)	7.58 (17.614)	-3.03 (14.213)	20.00 (28.109)	-6.67 (26.294)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-66.7, 33.3		
Week 8						
n	18	18	10	10		
Mean (SD)	12.96 (20.256)	0.00 (19.803)	20.00 (23.307)	-6.67 (37.843)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-100.0, 33.3		

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Modified Randomized Set

Appetite Loss

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	10		
Mean (SD)	5.00 (12.212)	-3.33 (18.417)	20.00 (23.307)	-6.67 (30.631)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-66.7, 33.3		
Week 24						
n	20	20	9	9		
Mean (SD)	6.67 (13.680)	-1.67 (22.878)	22.22 (28.868)	3.70 (20.031)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-33.3, 33.3		

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and Period 2 and Long Term Extension by Race
Modified Randomized Set

Appetite Loss

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	7	7	27	27
Mean (SD)	5.00 (12.212)	-1.67 (17.014)	14.29 (17.817)	0.00 (0.000)	7.41 (14.122)	-1.23 (14.563)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	0.0, 0.0	0.0, 33.3	-33.3, 33.3
Week 56						
n	16	16	5	5	21	21
Mean (SD)	8.33 (14.907)	0.00 (21.082)	26.67 (43.461)	13.33 (29.814)	12.70 (24.667)	3.17 (23.345)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	0.0, 66.7	0.0, 100.0	-33.3, 66.7

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Table GERMANY.2.5.4.9
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Modified Randomized Set

Appetite Loss

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	3	3	12	12
Mean (SD)	3.70 (11.111)	-7.41 (14.699)	22.22 (19.245)	0.00 (0.000)	8.33 (15.076)	-5.56 (12.975)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	0.0, 0.0	0.0, 33.3	-33.3, 0.0
Week 88						
n	7	7	2	2	9	9
Mean (SD)	9.52 (16.265)	-4.76 (12.599)	16.67 (23.570)	0.00 (0.000)	11.11 (16.667)	-3.70 (11.111)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	0.0, 0.0	0.0, 33.3	-33.3, 0.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Appetite Loss

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	0	0	2	2
Mean (SD)	0.00 (0.000)	0.00 (0.000)			0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Constipation

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	15.15 (26.681)		3.33 (10.541)		11.46 (23.355)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 33.3		0.0, 100.0	
Week 2						
n	22	22	10	10		
Mean (SD)	10.61 (25.996)	-4.55 (15.585)	16.67 (32.394)	13.33 (23.307)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	0.0, 66.7		

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Modified Randomized Set

Constipation

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	10		
Mean (SD)	12.12 (26.318)	-3.03 (14.213)	16.67 (32.394)	13.33 (23.307)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	0.0, 66.7		
Week 8						
n	18	18	10	10		
Mean (SD)	5.56 (17.150)	-11.11 (19.803)	6.67 (21.082)	3.33 (10.541)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 66.7	0.0, 33.3		

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Modified Randomized Set

Constipation

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	10		
Mean (SD)	10.00 (24.423)	-3.33 (18.417)	13.33 (32.203)	10.00 (22.498)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	0.0, 66.7		
Week 24						
n	20	20	9	9		
Mean (SD)	8.33 (23.878)	-5.00 (19.571)	18.52 (33.793)	14.81 (24.216)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	0.0, 66.7		

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Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1
and Period 2 and Long Term Extension by Race
Modified Randomized Set

Constipation

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	7	7	27	27
Mean (SD)	8.33 (14.809)	-6.67 (23.195)	4.76 (12.599)	4.76 (12.599)	7.41 (14.122)	-3.70 (21.350)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3	-66.7, 33.3
Week 56						
n	16	16	5	5	21	21
Mean (SD)	12.50 (26.874)	-4.17 (11.386)	0.00 (0.000)	0.00 (0.000)	9.52 (23.905)	-3.17 (10.026)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-33.3, 0.0	0.0, 0.0	0.0, 0.0	0.0, 100.0	-33.3, 0.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1
and Period 2 and Long Term Extension by Race
Modified Randomized Set

Constipation

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	3	3	12	12
Mean (SD)	14.81 (33.793)	-11.11 (16.667)	0.00 (0.000)	0.00 (0.000)	11.11 (29.588)	-8.33 (15.076)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-33.3, 0.0	0.0, 0.0	0.0, 0.0	0.0, 100.0	-33.3, 0.0
Week 88						
n	7	7	2	2	9	9
Mean (SD)	14.29 (17.817)	-4.76 (23.002)	0.00 (0.000)	0.00 (0.000)	11.11 (16.667)	-3.70 (20.031)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 0.0	0.0, 0.0	0.0, 33.3	-33.3, 33.3

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Constipation

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	0	0	2	2
Mean (SD)	16.67 (23.570)	0.00 (0.000)			16.67 (23.570)	0.00 (0.000)
Median	16.67	0.00			16.67	0.00
Min, Max	0.0, 33.3	0.0, 0.0			0.0, 33.3	0.0, 0.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Diarrhea

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	4.55 (21.320)		6.67 (14.055)		5.21 (19.138)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 33.3		0.0, 100.0	
Week 2						
n	22	22	10	10		
Mean (SD)	1.52 (7.107)	-3.03 (14.213)	10.00 (22.498)	3.33 (18.922)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 66.7	-33.3, 33.3		

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1
and Period 2 and Long Term Extension by Race
Modified Randomized Set

Diarrhea

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	10		
Mean (SD)	3.03 (9.808)	-1.52 (24.075)	6.67 (14.055)	0.00 (15.713)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	-33.3, 33.3		
Week 8						
n	18	18	10	10		
Mean (SD)	3.70 (10.779)	-1.85 (17.977)	3.33 (10.541)	-3.33 (18.922)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-33.3, 33.3		

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Table GERMANY.2.5.4.9
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Modified Randomized Set

Diarrhea

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	10		
Mean (SD)	5.00 (12.212)	0.00 (18.732)	3.33 (10.541)	-3.33 (18.922)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-33.3, 33.3		
Week 24						
n	20	20	9	9		
Mean (SD)	5.00 (12.212)	0.00 (18.732)	0.00 (0.000)	-3.70 (11.111)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 0.0	-33.3, 0.0		

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Table GERMANY.2.5.4.9
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and Period 2 and Long Term Extension by Race
Modified Randomized Set

Diarrhea

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	7	7	27	27
Mean (SD)	5.00 (12.212)	0.00 (26.491)	9.52 (16.265)	4.76 (12.599)	6.17 (13.195)	1.23 (23.537)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3	-100.0, 33.3
Week 56						
n	16	16	5	5	21	21
Mean (SD)	6.25 (13.437)	0.00 (29.814)	6.67 (14.907)	0.00 (0.000)	6.35 (13.412)	0.00 (25.820)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 33.3	0.0, 0.0	0.0, 33.3	-100.0, 33.3

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1
and Period 2 and Long Term Extension by Race
Modified Randomized Set

Diarrhea

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	3	3	12	12
Mean (SD)	0.00 (0.000)	0.00 (0.000)	11.11 (19.245)	0.00 (0.000)	2.78 (9.623)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 33.3	0.0, 0.0	0.0, 33.3	0.0, 0.0
Week 88						
n	7	7	2	2	9	9
Mean (SD)	0.00 (0.000)	0.00 (0.000)	16.67 (23.570)	0.00 (0.000)	3.70 (11.111)	0.00 (0.000)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 33.3	0.0, 0.0	0.0, 33.3	0.0, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Diarrhea

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	0	0	2	2
Mean (SD)	0.00 (0.000)	0.00 (0.000)			0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00			0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0			0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Financial Difficulties

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	25.76 (32.419)		16.67 (23.570)		22.92 (29.859)	
Median	16.67		0.00		0.00	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	22	22	10	10		
Mean (SD)	21.21 (28.257)	-4.55 (15.585)	16.67 (17.568)	0.00 (15.713)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 33.3	-33.3, 33.3		

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1
and Period 2 and Long Term Extension by Race
Modified Randomized Set

Financial Difficulties

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	10		
Mean (SD)	18.18 (22.366)	-7.58 (20.398)	10.00 (16.102)	-6.67 (21.082)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 33.3	-33.3, 33.3		
Week 8						
n	18	18	10	10		
Mean (SD)	12.96 (16.721)	-12.96 (30.548)	16.67 (23.570)	0.00 (22.222)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-100.0, 33.3	0.0, 66.7	-33.3, 33.3		

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Modified Randomized Set

Financial Difficulties

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	10		
Mean (SD)	18.33 (22.878)	-6.67 (23.195)	20.00 (28.109)	3.33 (29.187)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-33.3, 66.7		
Week 24						
n	20	20	9	9		
Mean (SD)	13.33 (19.941)	-11.67 (27.091)	14.81 (24.216)	-3.70 (20.031)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-100.0, 0.0	0.0, 66.7	-33.3, 33.3		

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Modified Randomized Set

Financial Difficulties

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	7	7	27	27
Mean (SD)	18.33 (27.519)	-10.00 (26.710)	9.52 (16.265)	-14.29 (17.817)	16.05 (25.099)	-11.11 (24.460)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-33.3, 0.0	0.0, 100.0	-66.7, 33.3
Week 56						
n	16	16	5	5	21	21
Mean (SD)	12.50 (20.638)	-14.58 (17.078)	13.33 (29.814)	-6.67 (27.889)	12.70 (22.301)	-12.70 (19.653)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3

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Modified Randomized Set

Financial Difficulties

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	3	3	12	12
Mean (SD)	3.70 (11.111)	-18.52 (29.397)	0.00 (0.000)	-22.22 (19.245)	2.78 (9.623)	-19.44 (26.432)
Median	0.00	0.00	0.00	-33.33	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 0.0	-33.3, 0.0	0.0, 33.3	-66.7, 0.0
Week 88						
n	7	7	2	2	9	9
Mean (SD)	19.05 (17.817)	-9.52 (16.265)	0.00 (0.000)	-33.33 (0.000)	14.81 (17.568)	-14.81 (17.568)
Median	33.33	0.00	0.00	-33.33	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 0.0	-33.3, -33.3	0.0, 33.3	-33.3, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Financial Difficulties

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	0	0	2	2
Mean (SD)	0.00 (0.000)	-33.33 (47.140)			0.00 (0.000)	-33.33 (47.140)
Median	0.00	-33.33			0.00	-33.33
Min, Max	0.0, 0.0	-66.7, 0.0			0.0, 0.0	-66.7, 0.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Fatigue

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		10		38	
Mean (SD)	44.05 (27.464)		50.00 (17.568)		45.61 (25.151)	
Median	44.44		44.44		44.44	
Min, Max	0.0, 100.0		33.3, 77.8		0.0, 100.0	
Week 2						
n	26	26	10	9		
Mean (SD)	32.05 (25.595)	-12.82 (21.703)	47.78 (21.628)	0.00 (14.699)		
Median	33.33	-11.11	38.89	0.00		
Min, Max	0.0, 88.9	-55.6, 33.3	33.3, 100.0	-22.2, 33.3		

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Modified Randomized Set

Fatigue

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	28	28	10	10		
Mean (SD)	26.39 (22.151)	-17.66 (22.984)	48.89 (19.738)	-1.11 (8.198)		
Median	22.22	-22.22	44.44	0.00		
Min, Max	0.0, 77.8	-55.6, 44.4	22.2, 77.8	-11.1, 11.1		
Week 8						
n	22	22	7	7		
Mean (SD)	28.28 (23.936)	-18.69 (27.727)	42.86 (18.624)	-3.17 (15.335)		
Median	27.78	-22.22	44.44	0.00		
Min, Max	0.0, 100.0	-66.7, 44.4	11.1, 66.7	-33.3, 11.1		

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Fatigue

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	25	25	10	10		
Mean (SD)	29.33 (19.479)	-15.11 (19.741)	42.22 (28.109)	-7.78 (19.633)		
Median	33.33	-11.11	44.44	-11.11		
Min, Max	0.0, 66.7	-55.6, 22.2	0.0, 100.0	-44.4, 22.2		
Week 24						
n	22	22	10	10		
Mean (SD)	25.25 (24.047)	-18.69 (27.300)	24.44 (19.458)	-25.56 (19.633)		
Median	22.22	-22.22	27.78	-27.78		
Min, Max	0.0, 88.9	-66.7, 33.3	0.0, 55.6	-55.6, 0.0		

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Modified Randomized Set

Fatigue

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	10	10	29	29
Mean (SD)	26.32 (24.063)	-15.79 (19.357)	41.11 (31.010)	-8.89 (23.888)	31.42 (27.065)	-13.41 (20.867)
Median	22.22	-11.11	33.33	-5.56	33.33	-11.11
Min, Max	0.0, 77.8	-55.6, 22.2	0.0, 100.0	-44.4, 33.3	0.0, 100.0	-55.6, 33.3
Week 56						
n	13	13	8	8	21	21
Mean (SD)	26.50 (22.008)	-10.26 (21.972)	33.33 (15.713)	-15.28 (17.755)	29.10 (19.713)	-12.17 (20.155)
Median	22.22	-11.11	27.78	-11.11	22.22	-11.11
Min, Max	0.0, 66.7	-33.3, 44.4	22.2, 66.7	-55.6, 0.0	0.0, 66.7	-55.6, 44.4

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Fatigue

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	6	6	15	15
Mean (SD)	30.86 (22.756)	-9.88 (16.144)	40.74 (16.728)	-5.56 (19.563)	34.81 (20.517)	-8.15 (17.042)
Median	33.33	-11.11	33.33	0.00	33.33	-11.11
Min, Max	0.0, 66.7	-33.3, 22.2	22.2, 66.7	-33.3, 22.2	0.0, 66.7	-33.3, 22.2
Week 88						
n	6	6	4	4	10	10
Mean (SD)	9.26 (12.989)	-20.37 (30.965)	41.67 (10.638)	-11.11 (20.286)	22.22 (20.286)	-16.67 (26.320)
Median	5.56	-33.33	38.89	-11.11	22.22	-27.78
Min, Max	0.0, 33.3	-44.4, 33.3	33.3, 55.6	-33.3, 11.1	0.0, 55.6	-44.4, 33.3

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Fatigue

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	33.33 (NA)	0.00 (NA)	16.67 (23.570)	0.00 (0.000)
Median	0.00	0.00	33.33	0.00	16.67	0.00
Min, Max	0.0, 0.0	0.0, 0.0	33.3, 33.3	0.0, 0.0	0.0, 33.3	0.0, 0.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Nausea and Vomiting

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		10		38	
Mean (SD)	2.98 (12.873)		0.00 (0.000)		2.19 (11.076)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 0.0		0.0, 66.7	
Week 2						
n	26	26	10	9		
Mean (SD)	5.13 (11.323)	1.92 (15.149)	6.67 (11.653)	3.70 (7.349)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 50.0	-50.0, 50.0	0.0, 33.3	0.0, 16.7		

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Modified Randomized Set

Nausea and Vomiting

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	28	28	10	10		
Mean (SD)	6.55 (11.421)	3.57 (14.598)	1.67 (5.270)	1.67 (5.270)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-50.0, 33.3	0.0, 16.7	0.0, 16.7		
Week 8						
n	22	22	7	7		
Mean (SD)	6.82 (19.009)	3.03 (19.678)	4.76 (8.133)	4.76 (8.133)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 83.3	-33.3, 83.3	0.0, 16.7	0.0, 16.7		

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Modified Randomized Set

Nausea and Vomiting

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	25	25	10	10		
Mean (SD)	2.67 (6.236)	-0.67 (11.263)	3.33 (7.027)	3.33 (7.027)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 16.7	-50.0, 16.7	0.0, 16.7	0.0, 16.7		
Week 24						
n	22	22	10	10		
Mean (SD)	3.03 (8.351)	-0.76 (14.065)	6.67 (8.607)	6.67 (8.607)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-50.0, 33.3	0.0, 16.7	0.0, 16.7		

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Modified Randomized Set

Nausea and Vomiting

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	10	10	29	29
Mean (SD)	5.26 (11.184)	5.26 (11.184)	3.33 (7.027)	3.33 (7.027)	4.60 (9.857)	4.60 (9.857)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 16.7	0.0, 16.7	0.0, 33.3	0.0, 33.3
Week 56						
n	13	13	8	8	21	21
Mean (SD)	1.28 (4.623)	1.28 (4.623)	6.25 (8.626)	6.25 (8.626)	3.17 (6.706)	3.17 (6.706)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 16.7	0.0, 16.7	0.0, 16.7	0.0, 16.7	0.0, 16.7	0.0, 16.7

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Modified Randomized Set

Nausea and Vomiting

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	6	6	15	15
Mean (SD)	1.85 (5.556)	1.85 (5.556)	8.33 (9.129)	8.33 (9.129)	4.44 (7.629)	4.44 (7.629)
Median	0.00	0.00	8.33	8.33	0.00	0.00
Min, Max	0.0, 16.7	0.0, 16.7	0.0, 16.7	0.0, 16.7	0.0, 16.7	0.0, 16.7
Week 88						
n	6	6	4	4	10	10
Mean (SD)	0.00 (0.000)	0.00 (0.000)	12.50 (8.333)	12.50 (8.333)	5.00 (8.051)	5.00 (8.051)
Median	0.00	0.00	16.67	16.67	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 16.7	0.0, 16.7	0.0, 16.7	0.0, 16.7

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Modified Randomized Set

Nausea and Vomiting

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	33.33 (NA)	33.33 (NA)	16.67 (23.570)	16.67 (23.570)
Median	0.00	0.00	33.33	33.33	16.67	16.67
Min, Max	0.0, 0.0	0.0, 0.0	33.3, 33.3	33.3, 33.3	0.0, 33.3	0.0, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Pain

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		10		38	
Mean (SD)	13.10 (23.288)		15.00 (19.954)		13.60 (22.211)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 83.3		0.0, 50.0		0.0, 83.3	
Week 2						
n	26	26	10	9		
Mean (SD)	12.18 (23.361)	-1.92 (18.455)	20.00 (18.922)	0.00 (16.667)		
Median	0.00	0.00	25.00	0.00		
Min, Max	0.0, 83.3	-33.3, 66.7	0.0, 50.0	-33.3, 33.3		

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Modified Randomized Set

Pain

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	28	28	10	10		
Mean (SD)	11.90 (20.213)	-1.19 (11.045)	11.67 (15.811)	-3.33 (13.147)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-16.7, 33.3	0.0, 33.3	-33.3, 16.7		
Week 8						
n	21	21	7	7		
Mean (SD)	11.11 (25.999)	1.59 (25.769)	9.52 (13.113)	-7.14 (13.113)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 33.3	-33.3, 0.0		

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Pain

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	25	25	10	10		
Mean (SD)	11.33 (19.076)	-3.33 (14.434)	8.33 (14.164)	-6.67 (21.082)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 16.7	0.0, 33.3	-33.3, 33.3		
Week 24						
n	22	22	10	10		
Mean (SD)	12.12 (19.370)	-3.03 (18.993)	6.67 (14.055)	-8.33 (14.164)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-50.0, 33.3	0.0, 33.3	-33.3, 0.0		

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and Period 2 and Long Term Extension by Race
Modified Randomized Set

Pain

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	10	10	29	29
Mean (SD)	12.28 (24.115)	0.00 (11.111)	21.67 (30.480)	6.67 (37.019)	15.52 (26.326)	2.30 (23.027)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 83.3	-33.3, 16.7	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0
Week 56						
n	13	13	8	8	21	21
Mean (SD)	12.82 (24.677)	-1.28 (12.659)	27.08 (23.465)	12.50 (27.817)	18.25 (24.667)	3.97 (20.348)
Median	0.00	0.00	25.00	8.33	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 16.7	0.0, 66.7	-16.7, 66.7	0.0, 66.7	-33.3, 66.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Pain

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	6	6	14	14
Mean (SD)	0.00 (0.000)	-4.17 (11.785)	25.00 (20.412)	11.11 (25.092)	10.71 (18.030)	2.38 (19.455)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 50.0	-16.7, 50.0	0.0, 50.0	-33.3, 50.0
Week 88						
n	6	6	4	4	10	10
Mean (SD)	0.00 (0.000)	0.00 (0.000)	25.00 (21.517)	25.00 (21.517)	10.00 (17.916)	10.00 (17.916)
Median	0.00	0.00	25.00	25.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 50.0	0.0, 50.0	0.0, 50.0	0.0, 50.0

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Modified Randomized Set

Pain

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	16.67 (NA)	16.67 (NA)	8.33 (11.785)	8.33 (11.785)
Median	0.00	0.00	16.67	16.67	8.33	8.33
Min, Max	0.0, 0.0	0.0, 0.0	16.7, 16.7	16.7, 16.7	0.0, 16.7	0.0, 16.7

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and Period 2 and Long Term Extension by Race
Modified Randomized Set

Dyspnoea

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		10		38	
Mean (SD)	35.71 (33.858)		26.67 (26.294)		33.33 (31.953)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	27	27	10	9		
Mean (SD)	23.46 (27.448)	-13.58 (24.909)	30.00 (29.187)	3.70 (20.031)		
Median	33.33	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 33.3		

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and Period 2 and Long Term Extension by Race
Modified Randomized Set

Dyspnoea

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	28	28	10	10		
Mean (SD)	15.48 (21.242)	-20.24 (27.725)	30.00 (39.907)	3.33 (29.187)		
Median	0.00	-33.33	16.67	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		
Week 8						
n	22	22	7	7		
Mean (SD)	19.70 (22.204)	-15.15 (28.595)	42.86 (31.706)	4.76 (29.991)		
Median	16.67	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-100.0, 33.3	0.0, 100.0	-33.3, 66.7		

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Modified Randomized Set

Dyspnoea

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	25	25	10	10		
Mean (SD)	18.67 (21.688)	-18.67 (27.352)	26.67 (21.082)	0.00 (27.217)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 66.7	-33.3, 33.3		
Week 24						
n	22	22	10	10		
Mean (SD)	15.15 (16.988)	-19.70 (28.469)	10.00 (16.102)	-16.67 (23.570)		
Median	0.00	-33.33	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 0.0		

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Dyspnoea

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	10	10	29	29
Mean (SD)	24.56 (26.856)	-14.04 (27.924)	13.33 (17.213)	-13.33 (17.213)	20.69 (24.257)	-13.79 (24.426)
Median	33.33	0.00	0.00	0.00	33.33	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-33.3, 0.0	0.0, 100.0	-66.7, 33.3
Week 56						
n	13	13	8	8	21	21
Mean (SD)	20.51 (25.598)	-15.38 (25.875)	12.50 (17.252)	-4.17 (11.785)	17.46 (22.655)	-11.11 (21.943)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-66.7, 33.3

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Modified Randomized Set

Dyspnoea

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	6	6	15	15
Mean (SD)	18.52 (24.216)	-18.52 (17.568)	16.67 (27.889)	-5.56 (32.773)	17.78 (24.774)	-13.33 (24.560)
Median	0.00	-33.33	0.00	-16.67	0.00	-33.33
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3
Week 88						
n	6	6	4	4	10	10
Mean (SD)	0.00 (0.000)	-16.67 (18.257)	16.67 (19.245)	0.00 (27.217)	6.67 (14.055)	-10.00 (22.498)
Median	0.00	-16.67	16.67	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3

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Modified Randomized Set

Dyspnoea

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	0.00 (NA)	0.00 (NA)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Insomnia

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		10		38	
Mean (SD)	30.95 (31.333)		33.33 (31.427)		31.58 (30.948)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	26	26	10	9		
Mean (SD)	24.36 (34.715)	-7.69 (21.721)	23.33 (31.623)	-11.11 (37.268)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 66.7		

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and Period 2 and Long Term Extension by Race
Modified Randomized Set

Insomnia

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	28	28	10	10		
Mean (SD)	28.57 (35.963)	-2.38 (23.880)	20.00 (23.307)	-13.33 (42.164)		
Median	0.00	0.00	16.67	-16.67		
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 66.7	-100.0, 33.3		
Week 8						
n	22	22	7	7		
Mean (SD)	19.70 (22.204)	-9.09 (25.577)	9.52 (16.265)	-19.05 (37.796)		
Median	16.67	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 33.3	0.0, 33.3	-100.0, 0.0		

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Insomnia

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	25	25	10	10		
Mean (SD)	21.33 (27.012)	-10.67 (20.905)	26.67 (40.976)	-6.67 (46.614)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 66.7		
Week 24						
n	22	22	10	10		
Mean (SD)	15.15 (26.681)	-15.15 (30.389)	20.00 (23.307)	-13.33 (28.109)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-100.0, 33.3	0.0, 66.7	-66.7, 33.3		

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Insomnia

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	10	10	29	29
Mean (SD)	28.07 (29.945)	-5.26 (22.942)	26.67 (34.427)	-6.67 (43.885)	27.59 (30.949)	-5.75 (30.949)
Median	33.33	0.00	16.67	0.00	33.33	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-100.0, 66.7	0.0, 100.0	-100.0, 66.7
Week 56						
n	13	13	8	8	21	21
Mean (SD)	20.51 (28.991)	-10.26 (25.036)	33.33 (30.861)	0.00 (25.198)	25.40 (29.636)	-6.35 (24.987)
Median	0.00	0.00	33.33	0.00	33.33	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-33.3, 33.3

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Modified Randomized Set

Insomnia

White

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	6	6	15	15
Mean (SD)	14.81 (24.216)	-11.11 (16.667)	27.78 (32.773)	-5.56 (49.065)	20.00 (27.603)	-8.89 (32.038)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 66.7	-100.0, 33.3	0.0, 66.7	-100.0, 33.3
Week 88						
n	6	6	4	4	10	10
Mean (SD)	0.00 (0.000)	-16.67 (18.257)	50.00 (43.033)	25.00 (31.914)	20.00 (35.832)	0.00 (31.427)
Median	0.00	-16.67	50.00	16.67	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 100.0	0.0, 66.7	0.0, 100.0	-33.3, 66.7

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Modified Randomized Set

Insomnia

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	100.00 (NA)	66.67 (NA)	50.00 (70.711)	33.33 (47.140)
Median	0.00	0.00	100.00	66.67	50.00	33.33
Min, Max	0.0, 0.0	0.0, 0.0	100.0, 100.0	66.7, 66.7	0.0, 100.0	0.0, 66.7

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Modified Randomized Set

Appetite Loss

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		10		38	
Mean (SD)	8.33 (14.699)		10.00 (16.102)		8.77 (14.875)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 33.3		0.0, 33.3		0.0, 33.3	
Week 2						
n	27	27	10	9		
Mean (SD)	12.35 (20.976)	3.70 (23.266)	16.67 (23.570)	3.70 (11.111)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	0.0, 33.3		

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Modified Randomized Set

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White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	28	28	10	10		
Mean (SD)	3.57 (10.499)	-4.76 (14.947)	13.33 (32.203)	3.33 (24.595)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-33.3, 66.7		
Week 8						
n	22	22	7	7		
Mean (SD)	13.64 (19.678)	3.03 (22.792)	4.76 (12.599)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 33.3	0.0, 0.0		

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Appetite Loss

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	25	25	10	10		
Mean (SD)	9.33 (24.570)	0.00 (30.429)	13.33 (28.109)	3.33 (18.922)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 66.7	-33.3, 33.3		
Week 24						
n	22	22	10	10		
Mean (SD)	13.64 (24.471)	4.55 (29.628)	10.00 (16.102)	0.00 (0.000)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 33.3	0.0, 0.0		

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Appetite Loss

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	10	10	29	29
Mean (SD)	7.02 (13.962)	-1.75 (17.476)	13.33 (23.307)	3.33 (18.922)	9.20 (17.586)	0.00 (17.817)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-33.3, 33.3
Week 56						
n	13	13	8	8	21	21
Mean (SD)	5.13 (12.518)	2.56 (16.452)	8.33 (15.430)	-4.17 (11.785)	6.35 (13.412)	0.00 (14.907)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 33.3

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Appetite Loss

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	6	6	15	15
Mean (SD)	3.70 (11.111)	0.00 (0.000)	11.11 (17.213)	0.00 (0.000)	6.67 (13.801)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 0.0	0.0, 33.3	0.0, 0.0	0.0, 33.3	0.0, 0.0
Week 88						
n	6	6	4	4	10	10
Mean (SD)	5.56 (13.608)	5.56 (13.608)	25.00 (16.667)	16.67 (19.245)	13.33 (17.213)	10.00 (16.102)
Median	0.00	0.00	33.33	16.67	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Appetite Loss

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	66.67 (NA)	33.33 (NA)	33.33 (47.140)	16.67 (23.570)
Median	0.00	0.00	66.67	33.33	33.33	16.67
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	33.3, 33.3	0.0, 66.7	0.0, 33.3

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1
and Period 2 and Long Term Extension by Race
Modified Randomized Set

Constipation

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		10		38	
Mean (SD)	9.52 (17.817)		13.33 (23.307)		10.53 (19.149)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 66.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	26	26	10	9		
Mean (SD)	7.69 (14.322)	-2.56 (18.674)	10.00 (22.498)	-3.70 (20.031)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 66.7	-33.3, 33.3		

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Modified Randomized Set

Constipation

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	28	28	10	10		
Mean (SD)	8.33 (19.510)	-1.19 (24.816)	13.33 (17.213)	0.00 (15.713)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 66.7	0.0, 33.3	-33.3, 33.3		
Week 8						
n	22	22	7	7		
Mean (SD)	7.58 (14.298)	0.00 (17.817)	23.81 (41.786)	9.52 (16.265)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	0.0, 33.3		

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Modified Randomized Set

Constipation

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	25	25	10	10		
Mean (SD)	5.33 (15.753)	-5.33 (22.933)	16.67 (28.328)	3.33 (10.541)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-66.7, 66.7	0.0, 66.7	0.0, 33.3		
Week 24						
n	22	22	10	10		
Mean (SD)	1.52 (7.107)	-7.58 (20.398)	6.67 (14.055)	-6.67 (14.055)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-33.3, 0.0		

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and Period 2 and Long Term Extension by Race
Modified Randomized Set

Constipation

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	10	10	29	29
Mean (SD)	0.00 (0.000)	-12.28 (19.909)	20.00 (28.109)	6.67 (26.294)	6.90 (18.643)	-5.75 (23.686)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-66.7, 66.7
Week 56						
n	13	13	8	8	21	21
Mean (SD)	5.13 (12.518)	-7.69 (24.167)	8.33 (15.430)	0.00 (0.000)	6.35 (13.412)	-4.76 (19.107)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-66.7, 33.3	0.0, 33.3	0.0, 0.0	0.0, 33.3	-66.7, 33.3

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and Period 2 and Long Term Extension by Race
Modified Randomized Set

Constipation

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	6	6	15	15
Mean (SD)	11.11 (16.667)	0.00 (23.570)	11.11 (17.213)	0.00 (21.082)	11.11 (16.265)	0.00 (21.822)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3
Week 88						
n	6	6	4	4	10	10
Mean (SD)	11.11 (17.213)	0.00 (21.082)	50.00 (57.735)	41.67 (50.000)	26.67 (40.976)	16.67 (39.284)
Median	0.00	0.00	50.00	33.33	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	0.0, 100.0	0.0, 100.0	-33.3, 100.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Constipation

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	66.67 (NA)	33.33 (NA)	33.33 (47.140)	16.67 (23.570)
Median	0.00	0.00	66.67	33.33	33.33	16.67
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	33.3, 33.3	0.0, 66.7	0.0, 33.3

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Diarrhea

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		10		38	
Mean (SD)	5.95 (13.001)		20.00 (28.109)		9.65 (18.837)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 33.3		0.0, 66.7		0.0, 66.7	
Week 2						
n	27	27	10	9		
Mean (SD)	4.94 (12.067)	-1.23 (6.415)	3.33 (10.541)	-18.52 (24.216)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-66.7, 0.0		

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Diarrhea

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	28	28	10	10		
Mean (SD)	4.76 (11.878)	-1.19 (14.293)	3.33 (10.541)	-16.67 (23.570)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-66.7, 0.0		
Week 8						
n	22	22	7	7		
Mean (SD)	10.61 (18.930)	6.06 (19.616)	14.29 (26.227)	-14.29 (17.817)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-33.3, 0.0		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Diarrhea

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	25	25	10	10		
Mean (SD)	5.33 (12.472)	-1.33 (11.706)	0.00 (0.000)	-20.00 (28.109)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 0.0	-66.7, 0.0		
Week 24						
n	22	22	10	10		
Mean (SD)	3.03 (9.808)	-1.52 (7.107)	10.00 (16.102)	-10.00 (22.498)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-66.7, 0.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Diarrhea

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	10	10	29	29
Mean (SD)	5.26 (12.488)	-3.51 (15.294)	6.67 (14.055)	-13.33 (28.109)	5.75 (12.814)	-6.90 (20.662)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3
Week 56						
n	13	13	8	8	21	21
Mean (SD)	7.69 (14.618)	2.56 (9.245)	8.33 (15.430)	-16.67 (30.861)	7.94 (14.548)	-4.76 (21.822)
Median	0.00	0.00	0.00	-16.67	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 33.3	-66.7, 33.3

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Table GERMANY.2.5.4.9
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Modified Randomized Set

Diarrhea

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	6	6	14	14
Mean (SD)	0.00 (0.000)	-4.17 (11.785)	38.89 (38.968)	5.56 (49.065)	16.67 (31.351)	0.00 (32.026)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0
Week 88						
n	6	6	4	4	10	10
Mean (SD)	0.00 (0.000)	0.00 (0.000)	0.00 (0.000)	-25.00 (31.914)	0.00 (0.000)	-10.00 (22.498)
Median	0.00	0.00	0.00	-16.67	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	-66.7, 0.0	0.0, 0.0	-66.7, 0.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Diarrhea

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	0.00 (NA)	0.00 (NA)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Financial Difficulties

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		10		38	
Mean (SD)	17.86 (30.741)		20.00 (23.307)		18.42 (28.682)	
Median	0.00		16.67		0.00	
Min, Max	0.0, 100.0		0.0, 66.7		0.0, 100.0	
Week 2						
n	28	28	10	9		
Mean (SD)	17.86 (33.311)	0.00 (15.713)	13.33 (23.307)	-3.70 (26.058)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 66.7	-66.7, 33.3		

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Modified Randomized Set

Financial Difficulties

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	28	28	10	10		
Mean (SD)	16.67 (30.765)	-1.19 (16.929)	6.67 (14.055)	-13.33 (23.307)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-66.7, 0.0		
Week 8						
n	22	22	7	7		
Mean (SD)	10.61 (23.874)	-3.03 (14.213)	14.29 (26.227)	0.00 (19.245)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 66.7	-33.3, 33.3		

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Financial Difficulties

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	25	25	10	10		
Mean (SD)	20.00 (31.914)	1.33 (11.706)	6.67 (14.055)	-13.33 (23.307)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 33.3	-66.7, 0.0		
Week 24						
n	22	22	10	10		
Mean (SD)	19.70 (33.585)	1.52 (12.503)	10.00 (16.102)	-10.00 (22.498)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 33.3	-66.7, 0.0		

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White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	10	10	29	29
Mean (SD)	17.54 (30.161)	-1.75 (20.709)	16.67 (23.570)	-3.33 (18.922)	17.24 (27.632)	-2.30 (19.783)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	-33.3, 33.3	0.0, 100.0	-66.7, 33.3
Week 56						
n	13	13	8	8	21	21
Mean (SD)	12.82 (28.991)	-2.56 (21.350)	16.67 (17.817)	-4.17 (27.817)	14.29 (24.881)	-3.17 (23.345)
Median	0.00	0.00	16.67	0.00	0.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 100.0	-66.7, 33.3

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Financial Difficulties

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	6	6	14	14
Mean (SD)	12.50 (17.252)	0.00 (17.817)	11.11 (17.213)	-11.11 (27.217)	11.90 (16.575)	-4.76 (22.100)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 33.3
Week 88						
n	6	6	4	4	10	10
Mean (SD)	0.00 (0.000)	-11.11 (27.217)	8.33 (16.667)	-16.67 (33.333)	3.33 (10.541)	-13.33 (28.109)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	-66.7, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties. Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem. Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan. D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point. Baseline is defined as the last non-missing value prior to the first dose of study drug. Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Financial Difficulties

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	0.00 (NA)	-66.67 (NA)	0.00 (0.000)	-33.33 (47.140)
Median	0.00	0.00	0.00	-66.67	0.00	-33.33
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	-66.7, -66.7	0.0, 0.0	-66.7, 0.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Fatigue

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		4		11	
Mean (SD)	57.14 (31.051)		50.00 (37.952)		54.55 (31.993)	
Median	55.56		44.44		55.56	
Min, Max	11.1, 100.0		11.1, 100.0		11.1, 100.0	
Week 2						
n	7	7	5	4		
Mean (SD)	44.44 (39.545)	-12.70 (33.597)	60.00 (32.011)	22.22 (20.286)		
Median	33.33	-22.22	66.67	22.22		
Min, Max	0.0, 100.0	-66.7, 33.3	11.1, 100.0	0.0, 44.4		

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Fatigue

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	7	7	5	4		
Mean (SD)	34.92 (33.597)	-22.22 (33.333)	53.33 (33.702)	13.89 (39.933)		
Median	33.33	-11.11	55.56	27.78		
Min, Max	0.0, 100.0	-66.7, 33.3	11.1, 100.0	-44.4, 44.4		
Week 8						
n	5	5	2	1		
Mean (SD)	28.89 (25.580)	-24.44 (21.373)	55.56 (31.427)	44.44 (NA)		
Median	33.33	-22.22	55.56	44.44		
Min, Max	0.0, 66.7	-55.6, 0.0	33.3, 77.8	44.4, 44.4		

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Fatigue

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	7	7	5	4		
Mean (SD)	33.33 (27.962)	-23.81 (14.947)	84.44 (18.592)	36.11 (41.944)		
Median	33.33	-22.22	88.89	33.33		
Min, Max	0.0, 66.7	-44.4, 0.0	55.6, 100.0	0.0, 77.8		
Week 24						
n	7	7	5	4		
Mean (SD)	38.10 (34.461)	-19.05 (30.574)	66.67 (28.328)	25.00 (47.467)		
Median	33.33	-22.22	66.67	27.78		
Min, Max	0.0, 100.0	-66.7, 33.3	33.3, 100.0	-33.3, 77.8		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Fatigue

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	7	7	4	4	11	11
Mean (SD)	42.86 (34.204)	-14.29 (31.239)	69.44 (35.573)	19.44 (53.190)	52.53 (35.518)	-2.02 (41.520)
Median	33.33	-11.11	72.22	33.33	44.44	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	33.3, 100.0	-55.6, 66.7	0.0, 100.0	-66.7, 66.7
Week 56						
n	5	5	4	3	9	8
Mean (SD)	46.67 (30.832)	-17.78 (40.521)	38.89 (11.111)	-14.81 (55.925)	43.21 (23.204)	-16.67 (42.828)
Median	33.33	-22.22	33.33	-22.22	33.33	-22.22
Min, Max	22.2, 100.0	-66.7, 33.3	33.3, 55.6	-66.7, 44.4	22.2, 100.0	-66.7, 44.4

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Fatigue

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	3	3	3	2	6	5
Mean (SD)	29.63 (27.962)	-37.04 (27.962)	66.67 (33.333)	16.67 (70.711)	48.15 (34.187)	-15.56 (50.062)
Median	33.33	-33.33	66.67	16.67	44.44	-33.33
Min, Max	0.0, 55.6	-66.7, -11.1	33.3, 100.0	-33.3, 66.7	0.0, 100.0	-66.7, 66.7
Week 88						
n	1	1	2	2	3	3
Mean (SD)	55.56 (NA)	-33.33 (NA)	55.56 (31.427)	-11.11 (78.567)	55.56 (22.222)	-18.52 (57.018)
Median	55.56	-33.33	55.56	-11.11	55.56	-33.33
Min, Max	55.6, 55.6	-33.3, -33.3	33.3, 77.8	-66.7, 44.4	33.3, 77.8	-66.7, 44.4

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Fatigue

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	33.33 (NA)	-55.56 (NA)	22.22 (NA)	-77.78 (NA)	27.78 (7.857)	-66.67 (15.713)
Median	33.33	-55.56	22.22	-77.78	27.78	-66.67
Min, Max	33.3, 33.3	-55.6, -55.6	22.2, 22.2	-77.8, -77.8	22.2, 33.3	-77.8, -55.6

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Nausea and Vomiting

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		4		11	
Mean (SD)	7.14 (13.113)		12.50 (15.957)		9.09 (13.670)	
Median	0.00		8.33		0.00	
Min, Max	0.0, 33.3		0.0, 33.3		0.0, 33.3	
Week 2						
n	7	7	5	4		
Mean (SD)	9.52 (18.898)	2.38 (26.227)	13.33 (18.257)	4.17 (8.333)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 50.0	-33.3, 50.0	0.0, 33.3	0.0, 16.7		

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Nausea and Vomiting

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	7	7	5	4		
Mean (SD)	4.76 (12.599)	-2.38 (20.250)	3.33 (7.454)	-8.33 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 16.7	-33.3, 0.0		
Week 8						
n	5	5	2	1		
Mean (SD)	6.67 (14.907)	-3.33 (24.721)	8.33 (11.785)	16.67 (NA)		
Median	0.00	0.00	8.33	16.67		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 16.7	16.7, 16.7		

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Nausea and Vomiting

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	7	7	5	4		
Mean (SD)	9.52 (16.265)	2.38 (14.996)	30.00 (36.132)	12.50 (28.464)		
Median	0.00	0.00	16.67	8.33		
Min, Max	0.0, 33.3	-16.7, 33.3	0.0, 83.3	-16.7, 50.0		
Week 24						
n	7	7	5	4		
Mean (SD)	11.90 (24.934)	4.76 (28.406)	3.33 (7.454)	-8.33 (21.517)		
Median	0.00	0.00	0.00	-8.33		
Min, Max	0.0, 66.7	-16.7, 66.7	0.0, 16.7	-33.3, 16.7		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties. Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem. Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan. D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point. Baseline is defined as the last non-missing value prior to the first dose of study drug. Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Nausea and Vomiting

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	7	7	4	4	11	11
Mean (SD)	14.29 (6.299)	7.14 (13.113)	16.67 (23.570)	4.17 (31.549)	15.15 (13.853)	6.06 (20.101)
Median	16.67	16.67	8.33	-8.33	16.67	0.00
Min, Max	0.0, 16.7	-16.7, 16.7	0.0, 50.0	-16.7, 50.0	0.0, 50.0	-16.7, 50.0
Week 56						
n	5	5	4	3	9	8
Mean (SD)	0.00 (0.000)	-10.00 (14.907)	25.00 (50.000)	16.67 (60.093)	11.11 (33.333)	0.00 (36.732)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 100.0	-33.3, 83.3	0.0, 100.0	-33.3, 83.3

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Nausea and Vomiting

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	3	3	3	2	6	5
Mean (SD)	0.00 (0.000)	-5.56 (9.623)	11.11 (19.245)	0.00 (47.140)	5.56 (13.608)	-3.33 (24.721)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	-16.7, 0.0	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3
Week 88						
n	1	1	2	2	3	3
Mean (SD)	0.00 (NA)	0.00 (NA)	0.00 (0.000)	-16.67 (23.570)	0.00 (0.000)	-11.11 (19.245)
Median	0.00	0.00	0.00	-16.67	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	-33.3, 0.0	0.0, 0.0	-33.3, 0.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Nausea and Vomiting

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	66.67 (NA)	33.33 (NA)	33.33 (47.140)	16.67 (23.570)
Median	0.00	0.00	66.67	33.33	33.33	16.67
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	33.3, 33.3	0.0, 66.7	0.0, 33.3

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Pain

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		4		11	
Mean (SD)	33.33 (38.490)		37.50 (41.667)		34.85 (37.605)	
Median	33.33		16.67		16.67	
Min, Max	0.0, 100.0		16.7, 100.0		0.0, 100.0	
Week 2						
n	7	7	4	3		
Mean (SD)	21.43 (36.911)	-11.90 (24.934)	45.83 (20.972)	11.11 (38.490)		
Median	0.00	0.00	50.00	33.33		
Min, Max	0.0, 100.0	-66.7, 0.0	16.7, 66.7	-33.3, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Pain

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	7	7	5	4		
Mean (SD)	28.57 (36.911)	-4.76 (20.893)	50.00 (40.825)	25.00 (41.944)		
Median	16.67	0.00	33.33	16.67		
Min, Max	0.0, 100.0	-50.0, 16.7	0.0, 100.0	-16.7, 83.3		
Week 8						
n	5	5	1	0		
Mean (SD)	36.67 (38.006)	10.00 (52.175)	16.67 (NA)			
Median	33.33	0.00	16.67			
Min, Max	0.0, 100.0	-33.3, 100.0	16.7, 16.7			

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Pain

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	7	7	4	3		
Mean (SD)	33.33 (43.033)	0.00 (33.333)	75.00 (28.868)	44.44 (53.576)		
Median	0.00	0.00	83.33	66.67		
Min, Max	0.0, 100.0	-66.7, 33.3	33.3, 100.0	-16.7, 83.3		
Week 24						
n	7	7	5	4		
Mean (SD)	33.33 (47.140)	0.00 (38.490)	23.33 (19.003)	-8.33 (41.944)		
Median	0.00	0.00	16.67	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 50.0	-66.7, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Pain

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	7	7	4	4	11	11
Mean (SD)	35.71 (35.262)	2.38 (29.547)	66.67 (38.490)	29.17 (36.956)	46.97 (37.873)	12.12 (33.409)
Median	16.67	0.00	66.67	16.67	33.33	16.67
Min, Max	0.0, 83.3	-50.0, 33.3	33.3, 100.0	0.0, 83.3	0.0, 100.0	-50.0, 83.3
Week 56						
n	5	5	4	3	9	8
Mean (SD)	40.00 (43.461)	-6.67 (36.515)	33.33 (23.570)	-5.56 (25.459)	37.04 (34.134)	-6.25 (30.780)
Median	33.33	0.00	25.00	0.00	33.33	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	16.7, 66.7	-33.3, 16.7	0.0, 100.0	-66.7, 33.3

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and Period 2 and Long Term Extension by Race
Modified Randomized Set

Pain

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	3	3	3	2	6	5
Mean (SD)	27.78 (34.694)	-27.78 (25.459)	38.89 (53.576)	33.33 (70.711)	33.33 (40.825)	-3.33 (51.908)
Median	16.67	-33.33	16.67	33.33	16.67	-16.67
Min, Max	0.0, 66.7	-50.0, 0.0	0.0, 100.0	-16.7, 83.3	0.0, 100.0	-50.0, 83.3
Week 88						
n	1	1	2	2	3	3
Mean (SD)	66.67 (NA)	-33.33 (NA)	50.00 (47.140)	33.33 (47.140)	55.56 (34.694)	11.11 (50.918)
Median	66.67	-33.33	50.00	33.33	66.67	0.00
Min, Max	66.7, 66.7	-33.3, -33.3	16.7, 83.3	0.0, 66.7	16.7, 83.3	-33.3, 66.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Pain

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	33.33 (NA)	-66.67 (NA)	16.67 (NA)	0.00 (NA)	25.00 (11.785)	-33.33 (47.140)
Median	33.33	-66.67	16.67	0.00	25.00	-33.33
Min, Max	33.3, 33.3	-66.7, -66.7	16.7, 16.7	0.0, 0.0	16.7, 33.3	-66.7, 0.0

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Modified Randomized Set

Dyspnoea

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		4		11	
Mean (SD)	38.10 (23.002)		33.33 (27.217)		36.36 (23.355)	
Median	33.33		33.33		33.33	
Min, Max	0.0, 66.7		0.0, 66.7		0.0, 66.7	
Week 2						
n	7	7	5	4		
Mean (SD)	23.81 (25.198)	-14.29 (17.817)	46.67 (29.814)	25.00 (41.944)		
Median	33.33	0.00	66.67	33.33		
Min, Max	0.0, 66.7	-33.3, 0.0	0.0, 66.7	-33.3, 66.7		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Dyspnoea

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	7	7	5	4		
Mean (SD)	9.52 (25.198)	-28.57 (23.002)	40.00 (43.461)	16.67 (43.033)		
Median	0.00	-33.33	33.33	16.67		
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 100.0	-33.3, 66.7		
Week 8						
n	5	5	2	1		
Mean (SD)	13.33 (18.257)	-20.00 (18.257)	33.33 (47.140)	33.33 (NA)		
Median	0.00	-33.33	33.33	33.33		
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 66.7	33.3, 33.3		

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Dyspnoea

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	7	7	5	4		
Mean (SD)	19.05 (26.227)	-19.05 (32.530)	53.33 (18.257)	25.00 (41.944)		
Median	0.00	-33.33	66.67	33.33		
Min, Max	0.0, 66.7	-66.7, 33.3	33.3, 66.7	-33.3, 66.7		
Week 24						
n	7	7	5	4		
Mean (SD)	23.81 (37.090)	-14.29 (37.796)	26.67 (14.907)	0.00 (27.217)		
Median	0.00	-33.33	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 33.3	-33.3, 33.3		

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and Period 2 and Long Term Extension by Race
Modified Randomized Set

Dyspnoea

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	7	7	4	4	11	11
Mean (SD)	23.81 (31.706)	-14.29 (17.817)	33.33 (0.000)	0.00 (27.217)	27.27 (25.025)	-9.09 (21.556)
Median	0.00	0.00	33.33	0.00	33.33	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	33.3, 33.3	-33.3, 33.3	0.0, 66.7	-33.3, 33.3
Week 56						
n	5	5	4	3	9	8
Mean (SD)	53.33 (38.006)	6.67 (27.889)	8.33 (16.667)	-22.22 (50.918)	33.33 (37.268)	-4.17 (37.533)
Median	66.67	0.00	0.00	-33.33	33.33	0.00
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 33.3	-66.7, 33.3	0.0, 100.0	-66.7, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Dyspnoea

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	3	3	3	2	6	5
Mean (SD)	22.22 (38.490)	-22.22 (19.245)	44.44 (19.245)	16.67 (23.570)	33.33 (29.814)	-6.67 (27.889)
Median	0.00	-33.33	33.33	16.67	33.33	0.00
Min, Max	0.0, 66.7	-33.3, 0.0	33.3, 66.7	0.0, 33.3	0.0, 66.7	-33.3, 33.3
Week 88						
n	1	1	2	2	3	3
Mean (SD)	0.00 (NA)	-66.67 (NA)	0.00 (0.000)	-33.33 (0.000)	0.00 (0.000)	-44.44 (19.245)
Median	0.00	-66.67	0.00	-33.33	0.00	-33.33
Min, Max	0.0, 0.0	-66.7, -66.7	0.0, 0.0	-33.3, -33.3	0.0, 0.0	-66.7, -33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Dyspnoea

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	-66.67 (NA)	33.33 (NA)	0.00 (NA)	16.67 (23.570)	-33.33 (47.140)
Median	0.00	-66.67	33.33	0.00	16.67	-33.33
Min, Max	0.0, 0.0	-66.7, -66.7	33.3, 33.3	0.0, 0.0	0.0, 33.3	-66.7, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Insomnia

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		4		11	
Mean (SD)	42.86 (25.198)		33.33 (47.140)		39.39 (32.722)	
Median	33.33		16.67		33.33	
Min, Max	0.0, 66.7		0.0, 100.0		0.0, 100.0	
Week 2						
n	7	7	5	4		
Mean (SD)	33.33 (38.490)	-9.52 (25.198)	46.67 (38.006)	16.67 (33.333)		
Median	33.33	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	0.0, 66.7		

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Insomnia

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	7	7	5	4		
Mean (SD)	23.81 (37.090)	-19.05 (37.796)	46.67 (38.006)	16.67 (43.033)		
Median	0.00	-33.33	33.33	16.67		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		
Week 8						
n	5	5	2	1		
Mean (SD)	13.33 (18.257)	-33.33 (33.333)	0.00 (0.000)	0.00 (NA)		
Median	0.00	-33.33	0.00	0.00		
Min, Max	0.0, 33.3	-66.7, 0.0	0.0, 0.0	0.0, 0.0		

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Insomnia

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	7	7	5	4		
Mean (SD)	28.57 (29.991)	-14.29 (26.227)	46.67 (38.006)	16.67 (19.245)		
Median	33.33	0.00	33.33	16.67		
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 100.0	0.0, 33.3		
Week 24						
n	7	7	5	4		
Mean (SD)	28.57 (35.635)	-14.29 (32.530)	26.67 (27.889)	-8.33 (16.667)		
Median	33.33	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 66.7	-33.3, 0.0		

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Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	7	7	4	4	11	11
Mean (SD)	33.33 (38.490)	-9.52 (31.706)	33.33 (47.140)	0.00 (54.433)	33.33 (39.441)	-6.06 (38.925)
Median	33.33	0.00	16.67	0.00	33.33	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7
Week 56						
n	5	5	4	3	9	8
Mean (SD)	40.00 (36.515)	-13.33 (29.814)	41.67 (41.944)	0.00 (66.667)	40.74 (36.430)	-8.33 (42.725)
Median	33.33	-33.33	33.33	0.00	33.33	-16.67
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-66.7, 66.7

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Insomnia

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	3	3	3	2	6	5
Mean (SD)	11.11 (19.245)	-44.44 (19.245)	55.56 (19.245)	16.67 (70.711)	33.33 (29.814)	-20.00 (50.553)
Median	0.00	-33.33	66.67	16.67	33.33	-33.33
Min, Max	0.0, 33.3	-66.7, -33.3	33.3, 66.7	-33.3, 66.7	0.0, 66.7	-66.7, 66.7
Week 88						
n	1	1	2	2	3	3
Mean (SD)	33.33 (NA)	-33.33 (NA)	16.67 (23.570)	-33.33 (47.140)	22.22 (19.245)	-33.33 (33.333)
Median	33.33	-33.33	16.67	-33.33	33.33	-33.33
Min, Max	33.3, 33.3	-33.3, -33.3	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Insomnia

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	-66.67 (NA)	0.00 (NA)	-100.00 (NA)	0.00 (0.000)	-83.33 (23.570)
Median	0.00	-66.67	0.00	-100.00	0.00	-83.33
Min, Max	0.0, 0.0	-66.7, -66.7	0.0, 0.0	-100.0, -100.0	0.0, 0.0	-100.0, -66.7

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Appetite Loss

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		4		11	
Mean (SD)	4.76 (12.599)		16.67 (33.333)		9.09 (21.556)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 33.3		0.0, 66.7		0.0, 66.7	
Week 2						
n	7	7	5	4		
Mean (SD)	9.52 (16.265)	4.76 (23.002)	20.00 (29.814)	8.33 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	0.0, 33.3		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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Appetite Loss

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	7	7	5	4		
Mean (SD)	9.52 (16.265)	4.76 (12.599)	0.00 (0.000)	-16.67 (33.333)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 0.0	-66.7, 0.0		
Week 8						
n	5	5	2	1		
Mean (SD)	13.33 (18.257)	6.67 (14.907)	0.00 (0.000)	0.00 (NA)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 0.0	0.0, 0.0		

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Appetite Loss

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	7	7	5	4		
Mean (SD)	4.76 (12.599)	0.00 (19.245)	20.00 (44.721)	8.33 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	0.0, 33.3		
Week 24						
n	7	7	5	4		
Mean (SD)	14.29 (37.796)	9.52 (41.786)	20.00 (29.814)	8.33 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 66.7	0.0, 33.3		

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1
and Period 2 and Long Term Extension by Race
Modified Randomized Set

Appetite Loss

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	7	7	4	4	11	11
Mean (SD)	9.52 (16.265)	4.76 (12.599)	41.67 (41.944)	25.00 (56.928)	21.21 (30.814)	12.12 (34.230)
Median	0.00	0.00	33.33	16.67	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 100.0	-33.3, 100.0	0.0, 100.0	-33.3, 100.0
Week 56						
n	5	5	4	3	9	8
Mean (SD)	13.33 (18.257)	6.67 (27.889)	0.00 (0.000)	-22.22 (38.490)	7.41 (14.699)	-4.17 (33.034)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 0.0	-66.7, 0.0	0.0, 33.3	-66.7, 33.3

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Appetite Loss

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	3	3	3	2	6	5
Mean (SD)	0.00 (0.000)	-11.11 (19.245)	33.33 (33.333)	16.67 (70.711)	16.67 (27.889)	0.00 (40.825)
Median	0.00	0.00	33.33	16.67	0.00	0.00
Min, Max	0.0, 0.0	-33.3, 0.0	0.0, 66.7	-33.3, 66.7	0.0, 66.7	-33.3, 66.7
Week 88						
n	1	1	2	2	3	3
Mean (SD)	0.00 (NA)	0.00 (NA)	0.00 (0.000)	-33.33 (47.140)	0.00 (0.000)	-22.22 (38.490)
Median	0.00	0.00	0.00	-33.33	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	-66.7, 0.0	0.0, 0.0	-66.7, 0.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Appetite Loss

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	66.67 (NA)	0.00 (NA)	33.33 (47.140)	0.00 (0.000)
Median	0.00	0.00	66.67	0.00	33.33	0.00
Min, Max	0.0, 0.0	0.0, 0.0	66.7, 66.7	0.0, 0.0	0.0, 66.7	0.0, 0.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Constipation

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		4		11	
Mean (SD)	23.81 (31.706)		25.00 (16.667)		24.24 (26.208)	
Median	0.00		33.33		33.33	
Min, Max	0.0, 66.7		0.0, 33.3		0.0, 66.7	
Week 2						
n	7	7	5	4		
Mean (SD)	14.29 (37.796)	-9.52 (31.706)	33.33 (40.825)	8.33 (41.944)		
Median	0.00	0.00	33.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 66.7		

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Constipation

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	7	7	5	4		
Mean (SD)	9.52 (25.198)	-14.29 (26.227)	33.33 (40.825)	16.67 (43.033)		
Median	0.00	0.00	33.33	16.67		
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 100.0	-33.3, 66.7		
Week 8						
n	5	5	2	1		
Mean (SD)	0.00 (0.000)	-20.00 (29.814)	33.33 (0.000)	33.33 (NA)		
Median	0.00	0.00	33.33	33.33		
Min, Max	0.0, 0.0	-66.7, 0.0	33.3, 33.3	33.3, 33.3		

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1
and Period 2 and Long Term Extension by Race
Modified Randomized Set

Constipation

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	7	7	5	4		
Mean (SD)	23.81 (41.786)	0.00 (19.245)	26.67 (43.461)	8.33 (41.944)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 66.7		
Week 24						
n	7	7	5	4		
Mean (SD)	19.05 (26.227)	-4.76 (23.002)	33.33 (23.570)	16.67 (19.245)		
Median	0.00	0.00	33.33	16.67		
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 66.7	0.0, 33.3		

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Constipation

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	7	7	4	4	11	11
Mean (SD)	19.05 (26.227)	-4.76 (23.002)	25.00 (16.667)	0.00 (27.217)	21.21 (22.473)	-3.03 (23.355)
Median	0.00	0.00	33.33	0.00	33.33	0.00
Min, Max	0.0, 66.7	-33.3, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-33.3, 33.3
Week 56						
n	5	5	4	3	9	8
Mean (SD)	13.33 (29.814)	-20.00 (29.814)	33.33 (27.217)	0.00 (33.333)	22.22 (28.868)	-12.50 (30.538)
Median	0.00	0.00	33.33	0.00	0.00	0.00
Min, Max	0.0, 66.7	-66.7, 0.0	0.0, 66.7	-33.3, 33.3	0.0, 66.7	-66.7, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Constipation

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	3	3	3	2	6	5
Mean (SD)	11.11 (19.245)	-22.22 (19.245)	22.22 (19.245)	16.67 (23.570)	16.67 (18.257)	-6.67 (27.889)
Median	0.00	-33.33	33.33	16.67	16.67	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 33.3	0.0, 33.3	0.0, 33.3	-33.3, 33.3
Week 88						
n	1	1	2	2	3	3
Mean (SD)	33.33 (NA)	-33.33 (NA)	0.00 (0.000)	-16.67 (23.570)	11.11 (19.245)	-22.22 (19.245)
Median	33.33	-33.33	0.00	-16.67	0.00	-33.33
Min, Max	33.3, 33.3	-33.3, -33.3	0.0, 0.0	-33.3, 0.0	0.0, 33.3	-33.3, 0.0

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Constipation

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	33.33 (NA)	-33.33 (NA)	0.00 (NA)	-33.33 (NA)	16.67 (23.570)	-33.33 (0.000)
Median	33.33	-33.33	0.00	-33.33	16.67	-33.33
Min, Max	33.3, 33.3	-33.3, -33.3	0.0, 0.0	-33.3, -33.3	0.0, 33.3	-33.3, -33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Diarrhea

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		4		11	
Mean (SD)	4.76 (12.599)		8.33 (16.667)		6.06 (13.484)	
Median	0.00		0.00		0.00	
Min, Max	0.0, 33.3		0.0, 33.3		0.0, 33.3	
Week 2						
n	7	7	5	4		
Mean (SD)	23.81 (41.786)	19.05 (37.796)	0.00 (0.000)	-8.33 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 100.0	0.0, 100.0	0.0, 0.0	-33.3, 0.0		

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Table GERMANY.2.5.4.9
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Diarrhea

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	7	7	5	4		
Mean (SD)	14.29 (26.227)	9.52 (25.198)	0.00 (0.000)	-8.33 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	0.0, 66.7	0.0, 0.0	-33.3, 0.0		
Week 8						
n	5	5	2	1		
Mean (SD)	6.67 (14.907)	0.00 (23.570)	0.00 (0.000)	0.00 (NA)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 0.0	0.0, 0.0		

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Diarrhea

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	7	7	5	4		
Mean (SD)	9.52 (16.265)	4.76 (23.002)	13.33 (29.814)	8.33 (41.944)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 66.7	-33.3, 66.7		
Week 24						
n	7	7	5	4		
Mean (SD)	14.29 (17.817)	9.52 (25.198)	0.00 (0.000)	-8.33 (16.667)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 0.0	-33.3, 0.0		

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Diarrhea

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	7	7	4	4	11	11
Mean (SD)	4.76 (12.599)	0.00 (19.245)	8.33 (16.667)	0.00 (27.217)	6.06 (13.484)	0.00 (21.082)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3	0.0, 33.3	-33.3, 33.3
Week 56						
n	5	5	4	3	9	8
Mean (SD)	6.67 (14.907)	6.67 (14.907)	8.33 (16.667)	-11.11 (19.245)	7.41 (14.699)	0.00 (17.817)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	0.0, 33.3	0.0, 33.3	-33.3, 0.0	0.0, 33.3	-33.3, 33.3

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Modified Randomized Set

Diarrhea

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	3	3	3	2	6	5
Mean (SD)	0.00 (0.000)	0.00 (0.000)	11.11 (19.245)	16.67 (23.570)	5.56 (13.608)	6.67 (14.907)
Median	0.00	0.00	0.00	16.67	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3
Week 88						
n	1	1	2	2	3	3
Mean (SD)	33.33 (NA)	33.33 (NA)	16.67 (23.570)	16.67 (23.570)	22.22 (19.245)	22.22 (19.245)
Median	33.33	33.33	16.67	16.67	33.33	33.33
Min, Max	33.3, 33.3	33.3, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3	0.0, 33.3

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Modified Randomized Set

Diarrhea

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	0.00 (NA)	0.00 (NA)	0.00 (0.000)	0.00 (0.000)
Median	0.00	0.00	0.00	0.00	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0	0.0, 0.0

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Financial Difficulties

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		4		11	
Mean (SD)	14.29 (26.227)		50.00 (57.735)		27.27 (41.682)	
Median	0.00		50.00		0.00	
Min, Max	0.0, 66.7		0.0, 100.0		0.0, 100.0	
Week 2						
n	7	7	5	4		
Mean (SD)	9.52 (16.265)	-4.76 (12.599)	60.00 (54.772)	25.00 (50.000)		
Median	0.00	0.00	100.00	0.00		
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 100.0	0.0, 100.0		

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Financial Difficulties

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	7	7	5	4		
Mean (SD)	19.05 (26.227)	4.76 (29.991)	60.00 (54.772)	25.00 (50.000)		
Median	0.00	0.00	100.00	0.00		
Min, Max	0.0, 66.7	-33.3, 66.7	0.0, 100.0	0.0, 100.0		
Week 8						
n	5	5	2	1		
Mean (SD)	20.00 (29.814)	0.00 (0.000)	0.00 (0.000)	0.00 (NA)		
Median	0.00	0.00	0.00	0.00		
Min, Max	0.0, 66.7	0.0, 0.0	0.0, 0.0	0.0, 0.0		

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Financial Difficulties

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	7	7	5	4		
Mean (SD)	9.52 (16.265)	-4.76 (12.599)	60.00 (54.772)	25.00 (50.000)		
Median	0.00	0.00	100.00	0.00		
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 100.0	0.0, 100.0		
Week 24						
n	7	7	5	4		
Mean (SD)	23.81 (37.090)	9.52 (41.786)	60.00 (54.772)	25.00 (50.000)		
Median	0.00	0.00	100.00	0.00		
Min, Max	0.0, 100.0	-33.3, 100.0	0.0, 100.0	0.0, 100.0		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Financial Difficulties

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	7	7	4	4	11	11
Mean (SD)	14.29 (17.817)	0.00 (19.245)	50.00 (57.735)	0.00 (81.650)	27.27 (38.925)	0.00 (47.140)
Median	0.00	0.00	50.00	0.00	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 33.3	0.0, 100.0	-100.0, 100.0	0.0, 100.0	-100.0, 100.0
Week 56						
n	5	5	4	3	9	8
Mean (SD)	13.33 (18.257)	-6.67 (14.907)	58.33 (50.000)	11.11 (83.887)	33.33 (40.825)	0.00 (47.140)
Median	0.00	0.00	66.67	0.00	33.33	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 100.0	-66.7, 100.0	0.0, 100.0	-66.7, 100.0

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Financial Difficulties

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	3	3	3	2	6	5
Mean (SD)	11.11 (19.245)	-11.11 (19.245)	22.22 (38.490)	-16.67 (23.570)	16.67 (27.889)	-13.33 (18.257)
Median	0.00	0.00	0.00	-16.67	0.00	0.00
Min, Max	0.0, 33.3	-33.3, 0.0	0.0, 66.7	-33.3, 0.0	0.0, 66.7	-33.3, 0.0
Week 88						
n	1	1	2	2	3	3
Mean (SD)	0.00 (NA)	0.00 (NA)	16.67 (23.570)	-33.33 (47.140)	11.11 (19.245)	-22.22 (38.490)
Median	0.00	0.00	16.67	-33.33	0.00	0.00
Min, Max	0.0, 0.0	0.0, 0.0	0.0, 33.3	-66.7, 0.0	0.0, 33.3	-66.7, 0.0

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.4.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Financial Difficulties

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.00 (NA)	0.00 (NA)	33.33 (NA)	-66.67 (NA)	16.67 (23.570)	-33.33 (47.140)
Median	0.00	0.00	33.33	-66.67	16.67	-33.33
Min, Max	0.0, 0.0	0.0, 0.0	33.3, 33.3	-66.7, -66.7	0.0, 33.3	-66.7, 0.0

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties. Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue
Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-12.39 (3.650)	-2.17 (5.795)	-10.21 (6.759)	0.1450
	95% CI for LS MEAN	(-19.94, -4.83)	(-14.16, 9.82)	(-24.23, 3.80)	
Week 4	LS Mean (SEM)	-13.94 (3.649)	0.39 (5.668)	-14.33 (6.658)	0.0415
	95% CI for LS MEAN	(-21.44, -6.44)	(-11.30, 12.07)	(-28.06, -0.60)	
Week 8	LS Mean (SEM)	-11.07 (2.804)	-10.97 (4.416)	-0.10 (5.091)	0.9844
	95% CI for LS MEAN	(-17.00, -5.14)	(-20.29, -1.65)	(-10.89, 10.69)	
Week 12	LS Mean (SEM)	-13.52 (3.889)	-2.08 (5.970)	-11.44 (7.046)	0.1155
	95% CI for LS MEAN	(-21.46, -5.58)	(-14.30, 10.13)	(-25.85, 2.98)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue
Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-12.00 (3.739)	1.63 (5.632)	-13.63 (6.662)	0.0474
	95% CI for LS MEAN	(-19.56, -4.44)	(-9.75, 13.01)	(-27.10, -0.17)	
Week 4	LS Mean (SEM)	-18.45 (3.792)	1.63 (5.751)	-20.08 (6.784)	0.0049
	95% CI for LS MEAN	(-26.08, -10.82)	(-9.94, 13.20)	(-33.73, -6.42)	
Week 8	LS Mean (SEM)	-19.25 (4.457)	3.30 (6.812)	-22.54 (8.011)	0.0079
	95% CI for LS MEAN	(-28.29, -10.21)	(-10.50, 17.09)	(-38.80, -6.29)	
Week 12	LS Mean (SEM)	-17.69 (3.961)	-0.59 (5.869)	-17.10 (6.977)	0.0184
	95% CI for LS MEAN	(-25.67, -9.71)	(-12.43, 11.24)	(-31.17, -3.03)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue

Subgroup by treatment interaction p-value	0.2249
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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting
Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.64 (0.877)	-0.70 (1.461)	0.06 (1.689)	0.9740
	95% CI for LS MEAN	(-2.38, 1.09)	(-3.60, 2.20)	(-3.29, 3.40)	
Week 4	LS Mean (SEM)	1.54 (0.860)	-0.65 (1.359)	2.19 (1.599)	0.1734
	95% CI for LS MEAN	(-0.17, 3.24)	(-3.35, 2.04)	(-0.98, 5.36)	
Week 8	LS Mean (SEM)	1.12 (0.952)	-0.67 (1.562)	1.79 (1.818)	0.3274
	95% CI for LS MEAN	(-0.77, 3.01)	(-3.77, 2.43)	(-1.82, 5.39)	
Week 12	LS Mean (SEM)	-0.63 (0.878)	-0.65 (1.359)	0.03 (1.610)	0.9873
	95% CI for LS MEAN	(-2.37, 1.11)	(-3.35, 2.04)	(-3.17, 3.22)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting
Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	1.39 (2.309)	3.46 (3.461)	-2.06 (4.126)	0.6194
	95% CI for LS MEAN	(-3.25, 6.04)	(-3.51, 10.42)	(-10.37, 6.24)	
Week 4	LS Mean (SEM)	0.37 (1.996)	-0.99 (3.021)	1.36 (3.576)	0.7053
	95% CI for LS MEAN	(-3.65, 4.40)	(-7.07, 5.10)	(-5.84, 8.57)	
Week 8	LS Mean (SEM)	1.77 (3.328)	6.51 (5.045)	-4.75 (5.996)	0.4339
	95% CI for LS MEAN	(-5.00, 8.53)	(-3.73, 16.75)	(-16.94, 7.44)	
Week 12	LS Mean (SEM)	3.17 (3.097)	3.46 (4.508)	-0.28 (5.438)	0.9586
	95% CI for LS MEAN	(-3.13, 9.48)	(-5.73, 12.65)	(-11.37, 10.80)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:03

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting

Subgroup by treatment interaction p-value 0.6362

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain
Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-5.07 (3.355)	1.70 (5.421)	-6.77 (6.256)	0.2906
	95% CI for LS MEAN	(-12.01, 1.87)	(-9.50, 12.90)	(-19.73, 6.19)	
Week 4	LS Mean (SEM)	-2.33 (3.195)	-2.68 (4.934)	0.35 (5.780)	0.9525
	95% CI for LS MEAN	(-8.86, 4.20)	(-12.77, 7.42)	(-11.49, 12.18)	
Week 8	LS Mean (SEM)	0.16 (5.533)	-6.18 (9.007)	6.35 (10.476)	0.5543
	95% CI for LS MEAN	(-11.59, 11.91)	(-25.50, 13.13)	(-16.11, 28.80)	
Week 12	LS Mean (SEM)	-8.47 (3.142)	1.03 (4.762)	-9.50 (5.614)	0.1055
	95% CI for LS MEAN	(-14.99, -1.96)	(-8.87, 10.93)	(-21.18, 2.18)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain
Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.27 (2.913)	5.05 (4.551)	-5.33 (5.367)	0.3261
	95% CI for LS MEAN	(-6.14, 5.59)	(-4.11, 14.21)	(-16.13, 5.47)	
Week 4	LS Mean (SEM)	-0.98 (3.127)	5.66 (4.791)	-6.64 (5.667)	0.2483
	95% CI for LS MEAN	(-7.29, 5.34)	(-4.01, 15.32)	(-18.08, 4.81)	
Week 8	LS Mean (SEM)	1.44 (4.601)	-1.56 (7.316)	3.00 (8.595)	0.7297
	95% CI for LS MEAN	(-7.98, 10.86)	(-16.51, 13.40)	(-14.59, 20.59)	
Week 12	LS Mean (SEM)	2.25 (4.031)	7.94 (6.081)	-5.68 (7.258)	0.4376
	95% CI for LS MEAN	(-5.86, 10.37)	(-4.30, 20.18)	(-20.30, 8.93)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain

Subgroup by treatment interaction p-value	0.6060
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea
Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-12.36 (3.786)	0.04 (6.057)	-12.39 (7.061)	0.0900
	95% CI for LS MEAN	(-20.10, -4.61)	(-12.34, 12.41)	(-26.85, 2.06)	
Week 4	LS Mean (SEM)	-18.33 (3.185)	-4.48 (4.912)	-13.86 (5.772)	0.0234
	95% CI for LS MEAN	(-24.85, -11.82)	(-14.55, 5.59)	(-25.69, -2.02)	
Week 8	LS Mean (SEM)	-12.43 (2.841)	-4.89 (4.430)	-7.54 (5.174)	0.1570
	95% CI for LS MEAN	(-18.27, -6.59)	(-13.99, 4.22)	(-18.18, 3.10)	
Week 12	LS Mean (SEM)	-15.48 (3.446)	-8.18 (5.292)	-7.29 (6.239)	0.2531
	95% CI for LS MEAN	(-22.55, -8.41)	(-19.07, 2.71)	(-20.13, 5.54)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea
Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-15.91 (3.881)	1.32 (6.052)	-17.23 (7.076)	0.0193
	95% CI for LS MEAN	(-23.74, -8.07)	(-10.89, 13.53)	(-31.52, -2.94)	
Week 4	LS Mean (SEM)	-24.73 (4.621)	3.55 (7.134)	-28.28 (8.405)	0.0015
	95% CI for LS MEAN	(-34.03, -15.43)	(-10.80, 17.89)	(-45.19, -11.36)	
Week 8	LS Mean (SEM)	-23.15 (5.016)	-5.42 (7.805)	-17.73 (9.190)	0.0602
	95% CI for LS MEAN	(-33.27, -13.03)	(-21.13, 10.30)	(-36.26, 0.80)	
Week 12	LS Mean (SEM)	-21.62 (4.632)	-3.12 (6.806)	-18.50 (8.137)	0.0284
	95% CI for LS MEAN	(-30.97, -12.27)	(-16.87, 10.63)	(-34.94, -2.06)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea

Subgroup by treatment interaction p-value	0.2263
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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Data cutoff date of 31Mar2023

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia
Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-6.35 (3.734)	-9.21 (5.867)	2.85 (6.855)	0.6805
	95% CI for LS MEAN	(-14.01, 1.30)	(-21.24, 2.82)	(-11.22, 16.93)	
Week 4	LS Mean (SEM)	-1.75 (4.321)	-9.63 (6.713)	7.89 (7.913)	0.3277
	95% CI for LS MEAN	(-10.59, 7.10)	(-23.41, 4.14)	(-8.35, 24.12)	
Week 8	LS Mean (SEM)	-5.31 (3.969)	-10.45 (6.326)	5.15 (7.351)	0.4903
	95% CI for LS MEAN	(-13.46, 2.85)	(-23.47, 2.56)	(-10.00, 20.30)	
Week 12	LS Mean (SEM)	-5.97 (3.644)	-9.63 (5.512)	3.66 (6.530)	0.5801
	95% CI for LS MEAN	(-13.47, 1.52)	(-21.00, 1.73)	(-9.80, 17.13)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia
Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.76 (4.597)	6.50 (6.934)	-9.26 (8.175)	0.2634
	95% CI for LS MEAN	(-12.03, 6.51)	(-7.47, 20.48)	(-25.75, 7.22)	
Week 4	LS Mean (SEM)	-7.17 (5.106)	2.06 (7.755)	-9.22 (9.150)	0.3187
	95% CI for LS MEAN	(-17.44, 3.11)	(-13.54, 17.65)	(-27.64, 9.19)	
Week 8	LS Mean (SEM)	-15.89 (4.740)	-0.80 (7.329)	-15.08 (8.544)	0.0869
	95% CI for LS MEAN	(-25.52, -6.25)	(-15.68, 14.07)	(-32.47, 2.31)	
Week 12	LS Mean (SEM)	-10.65 (4.424)	10.95 (6.461)	-21.60 (7.663)	0.0073
	95% CI for LS MEAN	(-19.57, -1.74)	(-2.09, 23.98)	(-37.06, -6.14)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia

Subgroup by treatment interaction p-value 0.0412

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss
Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.26 (3.728)	5.71 (5.807)	-5.97 (6.742)	0.3841
	95% CI for LS MEAN	(-7.91, 7.40)	(-6.22, 17.64)	(-19.85, 7.90)	
Week 4	LS Mean (SEM)	-1.84 (3.346)	6.00 (5.038)	-7.84 (5.901)	0.1949
	95% CI for LS MEAN	(-8.68, 5.01)	(-4.33, 16.33)	(-19.94, 4.26)	
Week 8	LS Mean (SEM)	2.77 (4.131)	7.02 (6.472)	-4.25 (7.545)	0.5839
	95% CI for LS MEAN	(-6.26, 11.80)	(-7.11, 21.15)	(-20.78, 12.28)	
Week 12	LS Mean (SEM)	-0.63 (5.447)	9.71 (8.457)	-10.34 (9.965)	0.3093
	95% CI for LS MEAN	(-11.80, 10.54)	(-7.68, 27.10)	(-30.83, 10.15)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss
Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.59 (2.988)	2.94 (4.563)	-4.52 (5.437)	0.4095
	95% CI for LS MEAN	(-7.60, 4.43)	(-6.24, 12.11)	(-15.46, 6.41)	
Week 4	LS Mean (SEM)	-7.47 (2.761)	-5.95 (4.227)	-1.52 (5.029)	0.7642
	95% CI for LS MEAN	(-13.03, -1.91)	(-14.45, 2.55)	(-11.63, 8.60)	
Week 8	LS Mean (SEM)	0.56 (4.201)	1.29 (6.454)	-0.72 (7.683)	0.9256
	95% CI for LS MEAN	(-7.93, 9.06)	(-11.74, 14.31)	(-16.25, 14.81)	
Week 12	LS Mean (SEM)	-4.31 (3.785)	-1.51 (5.524)	-2.80 (6.680)	0.6773
	95% CI for LS MEAN	(-11.93, 3.32)	(-12.65, 9.64)	(-16.27, 10.68)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss

Subgroup by treatment interaction p-value 0.3579

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation
Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.64 (2.562)	-6.26 (4.167)	5.62 (4.885)	0.2600
	95% CI for LS MEAN	(-5.89, 4.61)	(-14.79, 2.27)	(-4.39, 15.63)	
Week 4	LS Mean (SEM)	-2.64 (2.445)	-6.28 (3.870)	3.64 (4.573)	0.4319
	95% CI for LS MEAN	(-7.63, 2.35)	(-14.18, 1.62)	(-5.69, 12.98)	
Week 8	LS Mean (SEM)	-3.71 (2.553)	-6.35 (4.047)	2.64 (4.799)	0.5878
	95% CI for LS MEAN	(-8.99, 1.58)	(-14.72, 2.03)	(-7.31, 12.58)	
Week 12	LS Mean (SEM)	-5.60 (1.735)	-6.28 (2.709)	0.68 (3.218)	0.8336
	95% CI for LS MEAN	(-9.14, -2.05)	(-11.82, -0.74)	(-5.90, 7.26)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation
Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.41 (3.969)	12.92 (5.992)	-15.33 (7.072)	0.0354
	95% CI for LS MEAN	(-10.40, 5.57)	(0.86, 24.98)	(-29.57, -1.09)	
Week 4	LS Mean (SEM)	-0.31 (3.994)	17.36 (6.065)	-17.68 (7.144)	0.0171
	95% CI for LS MEAN	(-8.35, 7.72)	(5.16, 29.56)	(-32.06, -3.30)	
Week 8	LS Mean (SEM)	-5.91 (3.850)	16.03 (5.988)	-21.93 (6.934)	0.0032
	95% CI for LS MEAN	(-13.71, 1.90)	(3.90, 28.16)	(-36.01, -7.86)	
Week 12	LS Mean (SEM)	2.18 (4.001)	15.14 (5.838)	-12.96 (6.952)	0.0692
	95% CI for LS MEAN	(-5.88, 10.23)	(3.36, 26.92)	(-26.99, 1.06)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation

Subgroup by treatment interaction p-value	0.0497
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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea
Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	2.41 (2.763)	3.70 (5.009)	-1.28 (5.214)	0.8462
	95% CI for LS MEAN	(-32.70, 37.52)	(-59.95, 67.34)	(-67.53, 64.97)	
Week 4	LS Mean (SEM)	-1.93 (2.436)	3.23 (3.502)	-5.16 (3.912)	0.3654
	95% CI for LS MEAN	(-16.61, 12.75)	(-19.98, 26.44)	(-32.16, 21.84)	
Week 8	LS Mean (SEM)	2.74 (3.359)	3.23 (5.120)	-0.48 (5.895)	0.9386
	95% CI for LS MEAN	(-6.65, 12.14)	(-11.01, 17.47)	(-17.02, 16.05)	
Week 12	LS Mean (SEM)	0.92 (3.017)	3.23 (4.486)	-2.31 (5.133)	0.6838
	95% CI for LS MEAN	(-8.49, 10.33)	(-11.16, 17.62)	(-18.82, 14.20)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea
Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-3.38 (3.187)	-6.17 (4.796)	2.79 (5.761)	0.6299
	95% CI for LS MEAN	(-9.79, 3.04)	(-15.82, 3.47)	(-8.79, 14.38)	
Week 4	LS Mean (SEM)	-1.47 (2.701)	-8.39 (4.123)	6.93 (4.927)	0.1661
	95% CI for LS MEAN	(-6.90, 3.97)	(-16.68, -0.11)	(-2.98, 16.84)	
Week 8	LS Mean (SEM)	-0.59 (3.352)	-4.68 (5.274)	4.09 (6.235)	0.5155
	95% CI for LS MEAN	(-7.38, 6.21)	(-15.35, 5.99)	(-8.53, 16.72)	
Week 12	LS Mean (SEM)	-2.93 (2.793)	-8.39 (3.978)	5.47 (4.843)	0.2651
	95% CI for LS MEAN	(-8.56, 2.71)	(-16.41, -0.38)	(-4.30, 15.23)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea

Subgroup by treatment interaction p-value 0.1758

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties
Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	0.57 (4.006)	-7.31 (6.480)	7.87 (7.559)	0.3068
	95% CI for LS MEAN	(-7.65, 8.79)	(-20.59, 5.97)	(-7.64, 23.39)	
Week 4	LS Mean (SEM)	-6.68 (3.406)	-15.25 (5.332)	8.57 (6.268)	0.1819
	95% CI for LS MEAN	(-13.63, 0.27)	(-26.15, -4.35)	(-4.25, 21.39)	
Week 8	LS Mean (SEM)	-9.96 (3.723)	-10.46 (6.063)	0.50 (7.049)	0.9442
	95% CI for LS MEAN	(-17.60, -2.32)	(-22.90, 1.98)	(-13.98, 14.97)	
Week 12	LS Mean (SEM)	-2.40 (4.148)	-7.84 (6.491)	5.44 (7.651)	0.4827
	95% CI for LS MEAN	(-10.87, 6.07)	(-21.13, 5.44)	(-10.22, 21.10)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:03

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties
Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-4.27 (3.439)	9.96 (5.257)	-14.23 (6.116)	0.0247
	95% CI for LS MEAN	(-11.20, 2.66)	(-0.63, 20.55)	(-26.55, -1.90)	
Week 4	LS Mean (SEM)	-0.34 (3.920)	5.51 (5.971)	-5.86 (6.997)	0.4069
	95% CI for LS MEAN	(-8.24, 7.55)	(-6.51, 17.54)	(-19.96, 8.24)	
Week 8	LS Mean (SEM)	-4.42 (4.212)	10.81 (6.447)	-15.24 (7.571)	0.0554
	95% CI for LS MEAN	(-13.09, 4.25)	(-2.44, 24.06)	(-30.85, 0.38)	
Week 12	LS Mean (SEM)	-2.95 (3.953)	7.74 (5.904)	-10.69 (6.958)	0.1319
	95% CI for LS MEAN	(-10.91, 5.01)	(-4.17, 19.64)	(-24.72, 3.34)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.2
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties

Subgroup by treatment interaction p-value 0.0257

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue
< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-16.05 (3.196)	-2.78 (4.783)	-13.28 (5.635)	0.0223
	95% CI for LS MEAN	(-22.46, -9.64)	(-12.37, 6.81)	(-24.58, -1.97)	
Week 4	LS Mean (SEM)	-20.01 (3.253)	-2.78 (4.893)	-17.23 (5.757)	0.0041
	95% CI for LS MEAN	(-26.52, -13.49)	(-12.57, 7.02)	(-28.76, -5.70)	
Week 8	LS Mean (SEM)	-21.83 (3.091)	-6.22 (4.810)	-15.61 (5.587)	0.0082
	95% CI for LS MEAN	(-28.10, -15.57)	(-15.95, 3.51)	(-26.94, -4.29)	
Week 12	LS Mean (SEM)	-20.14 (3.294)	-5.86 (4.900)	-14.28 (5.787)	0.0167
	95% CI for LS MEAN	(-26.74, -13.55)	(-15.68, 3.95)	(-25.88, -2.68)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue
≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.32 (4.263)	9.31 (7.340)	-10.63 (8.478)	0.2267
	95% CI for LS MEAN	(-10.32, 7.68)	(-6.14, 24.76)	(-28.50, 7.24)	
Week 4	LS Mean (SEM)	-7.68 (4.463)	13.76 (7.148)	-21.44 (8.445)	0.0205
	95% CI for LS MEAN	(-17.04, 1.69)	(-1.26, 28.78)	(-39.17, -3.70)	
Week 8	LS Mean (SEM)	-4.48 (6.342)	5.96 (10.036)	-10.44 (11.901)	0.3956
	95% CI for LS MEAN	(-18.05, 9.09)	(-15.68, 27.61)	(-36.06, 15.17)	
Week 12	LS Mean (SEM)	-3.79 (5.563)	13.76 (8.496)	-17.55 (10.166)	0.1052
	95% CI for LS MEAN	(-15.59, 8.01)	(-4.43, 31.95)	(-39.24, 4.15)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue

Subgroup by treatment interaction p-value	0.6429
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting
< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.29 (1.679)	2.61 (2.503)	-2.90 (2.964)	0.3326
	95% CI for LS MEAN	(-3.65, 3.08)	(-2.40, 7.63)	(-8.84, 3.04)	
Week 4	LS Mean (SEM)	0.01 (1.604)	-1.09 (2.407)	1.10 (2.837)	0.7003
	95% CI for LS MEAN	(-3.21, 3.22)	(-5.92, 3.73)	(-4.59, 6.79)	
Week 8	LS Mean (SEM)	1.43 (2.903)	4.51 (4.494)	-3.08 (5.318)	0.5651
	95% CI for LS MEAN	(-4.44, 7.29)	(-4.56, 13.58)	(-13.83, 7.66)	
Week 12	LS Mean (SEM)	0.38 (2.204)	1.69 (3.270)	-1.31 (3.903)	0.7393
	95% CI for LS MEAN	(-4.07, 4.82)	(-4.92, 8.29)	(-9.19, 6.58)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting
≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	2.45 (2.273)	0.31 (3.772)	2.14 (4.406)	0.6290
	95% CI for LS MEAN	(-2.14, 7.05)	(-7.28, 7.90)	(-6.73, 11.02)	
Week 4	LS Mean (SEM)	2.46 (2.235)	0.53 (3.570)	1.92 (4.218)	0.6510
	95% CI for LS MEAN	(-2.07, 6.98)	(-6.68, 7.75)	(-6.61, 10.46)	
Week 8	LS Mean (SEM)	-1.14 (2.439)	3.90 (3.850)	-5.04 (4.540)	0.2730
	95% CI for LS MEAN	(-6.05, 3.77)	(-3.85, 11.65)	(-14.18, 4.10)	
Week 12	LS Mean (SEM)	3.55 (2.415)	3.31 (3.570)	0.23 (4.322)	0.9571
	95% CI for LS MEAN	(-1.32, 8.41)	(-3.91, 10.53)	(-8.49, 8.96)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting

Subgroup by treatment interaction p-value	0.4973
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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain
< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-4.46 (2.271)	4.17 (3.394)	-8.63 (3.967)	0.0340
	95% CI for LS MEAN	(-9.01, 0.09)	(-2.63, 10.97)	(-16.58, -0.68)	
Week 4	LS Mean (SEM)	-2.86 (2.811)	2.32 (4.224)	-5.17 (4.976)	0.3030
	95% CI for LS MEAN	(-8.49, 2.77)	(-6.15, 10.78)	(-15.15, 4.80)	
Week 8	LS Mean (SEM)	1.37 (4.544)	-3.11 (7.115)	4.47 (8.378)	0.5965
	95% CI for LS MEAN	(-7.83, 10.56)	(-17.49, 11.27)	(-12.48, 21.43)	
Week 12	LS Mean (SEM)	-2.44 (3.601)	4.17 (5.369)	-6.60 (6.389)	0.3059
	95% CI for LS MEAN	(-9.65, 4.77)	(-6.59, 14.93)	(-19.41, 6.21)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain
≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	3.34 (5.174)	3.19 (9.498)	0.15 (10.861)	0.9892
	95% CI for LS MEAN	(-7.50, 14.19)	(-16.67, 23.06)	(-22.57, 22.87)	
Week 4	LS Mean (SEM)	0.87 (3.305)	6.53 (5.211)	-5.66 (6.177)	0.3715
	95% CI for LS MEAN	(-6.05, 7.80)	(-4.41, 17.47)	(-18.62, 7.30)	
Week 8	LS Mean (SEM)	-2.37 (5.547)	-3.41 (9.401)	1.04 (10.947)	0.9257
	95% CI for LS MEAN	(-14.16, 9.41)	(-23.50, 16.67)	(-22.30, 24.38)	
Week 12	LS Mean (SEM)	-2.43 (4.573)	11.61 (7.704)	-14.04 (9.019)	0.1411
	95% CI for LS MEAN	(-12.13, 7.27)	(-4.93, 28.16)	(-33.31, 5.24)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain

Subgroup by treatment interaction p-value	0.5128
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Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea
< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-14.80 (3.605)	-0.44 (5.406)	-14.37 (6.342)	0.0278
	95% CI for LS MEAN	(-22.04, -7.57)	(-11.29, 10.41)	(-27.11, -1.63)	
Week 4	LS Mean (SEM)	-23.11 (3.770)	-0.44 (5.690)	-22.67 (6.671)	0.0013
	95% CI for LS MEAN	(-30.66, -15.56)	(-11.83, 10.95)	(-36.04, -9.31)	
Week 8	LS Mean (SEM)	-20.61 (3.677)	-7.69 (5.699)	-12.91 (6.621)	0.0572
	95% CI for LS MEAN	(-28.00, -13.21)	(-19.15, 3.76)	(-26.24, 0.41)	
Week 12	LS Mean (SEM)	-19.15 (3.776)	-5.99 (5.587)	-13.16 (6.583)	0.0511
	95% CI for LS MEAN	(-26.73, -11.58)	(-17.21, 5.22)	(-26.38, 0.06)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea
≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-13.35 (5.304)	7.98 (8.973)	-21.33 (10.420)	0.0553
	95% CI for LS MEAN	(-24.52, -2.18)	(-10.80, 26.77)	(-43.21, 0.54)	
Week 4	LS Mean (SEM)	-19.60 (5.869)	6.52 (9.461)	-26.12 (11.190)	0.0308
	95% CI for LS MEAN	(-31.89, -7.31)	(-13.31, 26.34)	(-49.55, -2.68)	
Week 8	LS Mean (SEM)	-14.98 (6.154)	0.47 (9.825)	-15.45 (11.700)	0.2039
	95% CI for LS MEAN	(-27.90, -2.06)	(-20.27, 21.21)	(-40.11, 9.21)	
Week 12	LS Mean (SEM)	-18.02 (5.153)	0.96 (7.795)	-18.98 (9.423)	0.0603
	95% CI for LS MEAN	(-28.85, -7.18)	(-15.56, 17.48)	(-38.87, 0.92)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea

Subgroup by treatment interaction p-value	0.7954
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Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia
< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-8.79 (3.777)	0.86 (5.657)	-9.65 (6.663)	0.1537
	95% CI for LS MEAN	(-16.37, -1.22)	(-10.50, 12.21)	(-23.03, 3.73)	
Week 4	LS Mean (SEM)	-6.23 (4.347)	-1.00 (6.554)	-5.24 (7.739)	0.5015
	95% CI for LS MEAN	(-14.94, 2.47)	(-14.12, 12.13)	(-20.74, 10.27)	
Week 8	LS Mean (SEM)	-18.08 (3.621)	-8.50 (5.641)	-9.58 (6.541)	0.1513
	95% CI for LS MEAN	(-25.40, -10.75)	(-19.90, 2.89)	(-22.81, 3.66)	
Week 12	LS Mean (SEM)	-11.48 (3.865)	4.56 (5.736)	-16.04 (6.774)	0.0214
	95% CI for LS MEAN	(-19.22, -3.74)	(-6.93, 16.05)	(-29.61, -2.47)	

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia
≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.82 (5.681)	6.70 (9.580)	0.12 (11.331)	0.9916
	95% CI for LS MEAN	(-5.21, 18.85)	(-13.47, 26.87)	(-23.70, 23.94)	
Week 4	LS Mean (SEM)	-3.04 (5.170)	-1.51 (8.119)	-1.53 (9.817)	0.8781
	95% CI for LS MEAN	(-13.87, 7.80)	(-18.58, 15.57)	(-22.11, 19.06)	
Week 8	LS Mean (SEM)	1.93 (5.661)	6.81 (8.742)	-4.88 (10.539)	0.6491
	95% CI for LS MEAN	(-10.02, 13.88)	(-11.61, 25.23)	(-27.13, 17.36)	
Week 12	LS Mean (SEM)	-3.11 (6.032)	4.05 (8.573)	-7.15 (10.672)	0.5123
	95% CI for LS MEAN	(-15.84, 9.63)	(-14.23, 22.32)	(-29.77, 15.47)	

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia

Subgroup by treatment interaction p-value	0.2573
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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss
< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.72 (2.578)	4.38 (3.948)	-7.11 (4.675)	0.1341
	95% CI for LS MEAN	(-7.89, 2.44)	(-3.52, 12.29)	(-16.47, 2.26)	
Week 4	LS Mean (SEM)	-5.32 (2.877)	-1.17 (4.404)	-4.14 (5.221)	0.4306
	95% CI for LS MEAN	(-11.08, 0.44)	(-9.99, 7.64)	(-14.59, 6.31)	
Week 8	LS Mean (SEM)	-1.13 (3.248)	-0.78 (5.143)	-0.35 (6.031)	0.9546
	95% CI for LS MEAN	(-7.73, 5.48)	(-11.20, 9.64)	(-12.59, 11.90)	
Week 12	LS Mean (SEM)	-3.84 (3.434)	0.68 (5.171)	-4.52 (6.173)	0.4670
	95% CI for LS MEAN	(-10.72, 3.03)	(-9.67, 11.03)	(-16.88, 7.84)	

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss
≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.37 (4.880)	6.86 (8.647)	-1.49 (9.927)	0.8819
	95% CI for LS MEAN	(-4.86, 15.59)	(-11.19, 24.91)	(-22.24, 19.25)	
Week 4	LS Mean (SEM)	-2.97 (2.453)	0.45 (3.913)	-3.42 (4.622)	0.4693
	95% CI for LS MEAN	(-8.12, 2.19)	(-7.79, 8.69)	(-13.14, 6.31)	
Week 8	LS Mean (SEM)	8.37 (5.897)	4.19 (9.522)	4.17 (11.209)	0.7136
	95% CI for LS MEAN	(-3.94, 20.68)	(-15.70, 24.09)	(-19.24, 27.59)	
Week 12	LS Mean (SEM)	0.38 (6.428)	11.56 (9.941)	-11.19 (11.842)	0.3567
	95% CI for LS MEAN	(-13.04, 13.79)	(-9.27, 32.40)	(-35.97, 13.60)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss

Subgroup by treatment interaction p-value	0.6095
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation
< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.46 (3.405)	10.07 (5.116)	-11.53 (5.999)	0.0598
	95% CI for LS MEAN	(-8.29, 5.36)	(-0.18, 20.32)	(-23.56, 0.49)	
Week 4	LS Mean (SEM)	-1.23 (3.507)	10.07 (5.286)	-11.30 (6.198)	0.0736
	95% CI for LS MEAN	(-8.25, 5.79)	(-0.51, 20.65)	(-23.72, 1.12)	
Week 8	LS Mean (SEM)	-6.48 (3.171)	10.00 (4.941)	-16.48 (5.709)	0.0061
	95% CI for LS MEAN	(-12.87, -0.08)	(0.05, 19.95)	(-27.99, -4.97)	
Week 12	LS Mean (SEM)	-0.81 (3.412)	11.92 (5.072)	-12.72 (5.962)	0.0374
	95% CI for LS MEAN	(-7.64, 6.03)	(1.76, 22.08)	(-24.68, -0.77)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation
≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-3.02 (4.805)	-2.81 (7.997)	-0.20 (9.561)	0.9832
	95% CI for LS MEAN	(-13.19, 7.15)	(-19.67, 14.04)	(-20.36, 19.95)	
Week 4	LS Mean (SEM)	-3.16 (5.232)	8.14 (8.570)	-11.30 (10.261)	0.2871
	95% CI for LS MEAN	(-14.27, 7.95)	(-10.05, 26.33)	(-33.03, 10.44)	
Week 8	LS Mean (SEM)	-0.46 (5.713)	2.99 (9.227)	-3.45 (11.095)	0.7604
	95% CI for LS MEAN	(-12.66, 11.74)	(-16.77, 22.75)	(-27.16, 20.27)	
Week 12	LS Mean (SEM)	-5.09 (4.513)	-2.97 (7.211)	-2.12 (8.800)	0.8126
	95% CI for LS MEAN	(-14.59, 4.41)	(-18.24, 12.29)	(-20.68, 16.44)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation

Subgroup by treatment interaction p-value	0.2636
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea
< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.38 (2.920)	-5.82 (4.406)	4.44 (5.258)	0.4022
	95% CI for LS MEAN	(-7.23, 4.47)	(-14.64, 3.01)	(-6.10, 14.98)	
Week 4	LS Mean (SEM)	-3.01 (2.236)	-7.67 (3.374)	4.67 (4.012)	0.2499
	95% CI for LS MEAN	(-7.48, 1.47)	(-14.43, -0.92)	(-3.37, 12.70)	
Week 8	LS Mean (SEM)	1.36 (2.993)	-3.72 (4.655)	5.08 (5.508)	0.3607
	95% CI for LS MEAN	(-4.66, 7.39)	(-13.08, 5.63)	(-6.00, 16.17)	
Week 12	LS Mean (SEM)	-4.09 (1.558)	-11.37 (2.302)	7.28 (2.724)	0.0101
	95% CI for LS MEAN	(-7.22, -0.96)	(-16.00, -6.75)	(1.81, 12.76)	

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Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea
≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.16 (3.105)	6.90 (5.270)	-7.06 (6.112)	0.2529
	95% CI for LS MEAN	(-6.39, 6.07)	(-3.65, 17.44)	(-19.29, 5.18)	
Week 4	LS Mean (SEM)	2.20 (3.025)	6.69 (4.844)	-4.49 (5.718)	0.4359
	95% CI for LS MEAN	(-3.88, 8.28)	(-3.03, 16.42)	(-15.97, 6.99)	
Week 8	LS Mean (SEM)	0.27 (3.392)	7.87 (5.343)	-7.59 (6.296)	0.2329
	95% CI for LS MEAN	(-6.52, 7.07)	(-2.83, 18.57)	(-20.21, 5.02)	
Week 12	LS Mean (SEM)	7.64 (3.353)	17.80 (4.844)	-10.16 (5.907)	0.0914
	95% CI for LS MEAN	(0.92, 14.36)	(8.08, 27.53)	(-22.01, 1.69)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea

Subgroup by treatment interaction p-value	0.0107
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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties
< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	0.25 (3.350)	8.37 (5.048)	-8.12 (5.886)	0.1735
	95% CI for LS MEAN	(-6.46, 6.97)	(-1.74, 18.49)	(-19.93, 3.69)	
Week 4	LS Mean (SEM)	-2.19 (3.691)	0.97 (5.564)	-3.15 (6.522)	0.6307
	95% CI for LS MEAN	(-9.58, 5.21)	(-10.18, 12.11)	(-16.22, 9.92)	
Week 8	LS Mean (SEM)	-7.40 (3.626)	5.06 (5.638)	-12.46 (6.545)	0.0649
	95% CI for LS MEAN	(-14.75, -0.04)	(-6.35, 16.47)	(-25.73, 0.81)	
Week 12	LS Mean (SEM)	-2.39 (3.693)	6.52 (5.525)	-8.91 (6.489)	0.1751
	95% CI for LS MEAN	(-9.78, 5.00)	(-4.54, 17.58)	(-21.91, 4.09)	

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Table GERMANY.2.5.2.3
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties
≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-6.42 (2.448)	-7.14 (4.304)	0.72 (4.945)	0.8852
	95% CI for LS MEAN	(-11.59, -1.25)	(-16.19, 1.90)	(-9.68, 11.13)	
Week 4	LS Mean (SEM)	-2.25 (2.847)	-9.45 (4.627)	7.20 (5.435)	0.2019
	95% CI for LS MEAN	(-8.23, 3.73)	(-19.17, 0.27)	(-4.22, 18.61)	
Week 8	LS Mean (SEM)	-3.26 (2.780)	-8.21 (4.591)	4.94 (5.355)	0.3676
	95% CI for LS MEAN	(-9.08, 2.55)	(-17.80, 1.39)	(-6.26, 16.14)	
Week 12	LS Mean (SEM)	-0.27 (2.758)	-9.45 (4.407)	9.18 (5.201)	0.0957
	95% CI for LS MEAN	(-6.07, 5.54)	(-18.77, -0.13)	(-1.80, 20.17)	

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties

Subgroup by treatment interaction p-value	0.1347
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:48:06

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue
Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-10.31 (4.397)	4.27 (6.409)	-14.58 (7.698)	0.0675
	95% CI for LS MEAN	(-19.28, -1.34)	(-8.80, 17.34)	(-30.28, 1.12)	
Week 4	LS Mean (SEM)	-15.80 (4.485)	2.42 (6.614)	-18.22 (7.908)	0.0274
	95% CI for LS MEAN	(-24.92, -6.69)	(-11.01, 15.85)	(-34.29, -2.15)	
Week 8	LS Mean (SEM)	-19.19 (5.136)	11.44 (8.272)	-30.63 (9.640)	0.0038
	95% CI for LS MEAN	(-29.78, -8.60)	(-5.52, 28.39)	(-50.44, -10.81)	
Week 12	LS Mean (SEM)	-14.77 (4.662)	7.97 (6.706)	-22.75 (8.081)	0.0086
	95% CI for LS MEAN	(-24.30, -5.25)	(-5.73, 21.67)	(-39.27, -6.22)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:08

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue
Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-13.02 (3.123)	-3.93 (5.078)	-9.09 (5.836)	0.1279
	95% CI for LS MEAN	(-19.34, -6.70)	(-14.20, 6.34)	(-20.92, 2.74)	
Week 4	LS Mean (SEM)	-16.64 (3.126)	0.21 (4.943)	-16.85 (5.739)	0.0055
	95% CI for LS MEAN	(-22.95, -10.33)	(-9.77, 10.20)	(-28.45, -5.26)	
Week 8	LS Mean (SEM)	-13.08 (3.110)	-12.18 (4.718)	-0.90 (5.507)	0.8717
	95% CI for LS MEAN	(-19.37, -6.78)	(-21.75, -2.61)	(-12.09, 10.29)	
Week 12	LS Mean (SEM)	-16.46 (3.057)	-9.97 (4.712)	-6.49 (5.504)	0.2454
	95% CI for LS MEAN	(-22.63, -10.30)	(-19.50, -0.44)	(-17.62, 4.64)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:08

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue

Subgroup by treatment interaction p-value	0.0717
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:08

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting
Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.50 (2.536)	2.57 (3.657)	2.93 (4.463)	0.5164
	95% CI for LS MEAN	(0.35, 10.64)	(-4.85, 9.99)	(-6.13, 11.98)	
Week 4	LS Mean (SEM)	6.15 (2.388)	-1.60 (3.488)	7.74 (4.239)	0.0777
	95% CI for LS MEAN	(1.27, 11.02)	(-8.71, 5.52)	(-0.91, 16.39)	
Week 8	LS Mean (SEM)	-0.15 (1.404)	7.91 (2.316)	-8.06 (2.640)	0.0112
	95% CI for LS MEAN	(-3.32, 3.01)	(2.89, 12.93)	(-13.89, -2.24)	
Week 12	LS Mean (SEM)	3.19 (2.453)	6.74 (3.477)	-3.54 (4.266)	0.4131
	95% CI for LS MEAN	(-1.83, 8.22)	(-0.39, 13.86)	(-12.28, 5.20)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:08

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting
Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.92 (1.313)	0.13 (2.203)	-3.05 (2.540)	0.2377
	95% CI for LS MEAN	(-5.58, -0.27)	(-4.33, 4.59)	(-8.19, 2.10)	
Week 4	LS Mean (SEM)	-2.61 (1.221)	-1.87 (1.963)	-0.73 (2.294)	0.7517
	95% CI for LS MEAN	(-5.07, -0.14)	(-5.84, 2.09)	(-5.36, 3.90)	
Week 8	LS Mean (SEM)	3.04 (3.340)	-0.35 (5.247)	3.38 (6.218)	0.5898
	95% CI for LS MEAN	(-3.73, 9.80)	(-11.00, 10.31)	(-9.23, 16.00)	
Week 12	LS Mean (SEM)	-0.80 (1.514)	-4.65 (2.352)	3.85 (2.784)	0.1769
	95% CI for LS MEAN	(-3.88, 2.28)	(-9.46, 0.15)	(-1.83, 9.53)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:08

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting

Subgroup by treatment interaction p-value	0.7822
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain
Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.21 (3.688)	4.50 (5.501)	-6.71 (6.613)	0.3173
	95% CI for LS MEAN	(-9.71, 5.29)	(-6.67, 15.68)	(-20.15, 6.73)	
Week 4	LS Mean (SEM)	-0.79 (3.821)	6.34 (5.571)	-7.13 (6.737)	0.2977
	95% CI for LS MEAN	(-8.58, 6.99)	(-5.00, 17.68)	(-20.85, 6.59)	
Week 8	LS Mean (SEM)	-8.37 (1.665)	-7.78 (2.919)	-0.59 (3.313)	0.8616
	95% CI for LS MEAN	(-12.02, -4.72)	(-14.09, -1.46)	(-7.80, 6.62)	
Week 12	LS Mean (SEM)	-3.31 (4.890)	7.83 (7.125)	-11.13 (8.629)	0.2057
	95% CI for LS MEAN	(-13.25, 6.64)	(-6.65, 22.31)	(-28.67, 6.41)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:08

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain
Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.73 (2.622)	3.44 (4.248)	-6.16 (4.873)	0.2136
	95% CI for LS MEAN	(-8.02, 2.57)	(-5.14, 12.01)	(-16.01, 3.69)	
Week 4	LS Mean (SEM)	-2.93 (2.668)	-1.44 (4.176)	-1.49 (4.857)	0.7610
	95% CI for LS MEAN	(-8.31, 2.45)	(-9.87, 6.99)	(-11.30, 8.32)	
Week 8	LS Mean (SEM)	5.09 (5.402)	-5.74 (8.165)	10.83 (9.723)	0.2733
	95% CI for LS MEAN	(-5.87, 16.05)	(-22.35, 10.86)	(-8.95, 30.61)	
Week 12	LS Mean (SEM)	-2.65 (3.339)	2.72 (5.133)	-5.37 (6.044)	0.3800
	95% CI for LS MEAN	(-9.39, 4.09)	(-7.67, 13.12)	(-17.61, 6.87)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain

Subgroup by treatment interaction p-value	0.2947
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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea
Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-13.85 (4.276)	3.41 (6.417)	-17.26 (7.664)	0.0313
	95% CI for LS MEAN	(-22.57, -5.13)	(-9.65, 16.47)	(-32.87, -1.65)	
Week 4	LS Mean (SEM)	-21.85 (4.834)	0.63 (7.194)	-22.48 (8.626)	0.0134
	95% CI for LS MEAN	(-31.67, -12.03)	(-13.96, 15.23)	(-40.00, -4.97)	
Week 8	LS Mean (SEM)	-14.20 (4.870)	8.69 (7.904)	-22.88 (9.223)	0.0206
	95% CI for LS MEAN	(-24.30, -4.09)	(-7.57, 24.94)	(-41.93, -3.83)	
Week 12	LS Mean (SEM)	-15.92 (4.590)	6.19 (6.624)	-22.11 (8.014)	0.0097
	95% CI for LS MEAN	(-25.29, -6.56)	(-7.32, 19.70)	(-38.47, -5.76)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea
Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-14.33 (4.315)	-1.25 (6.961)	-13.08 (8.024)	0.1119
	95% CI for LS MEAN	(-23.08, -5.59)	(-15.35, 12.86)	(-29.37, 3.20)	
Week 4	LS Mean (SEM)	-21.85 (4.158)	1.06 (6.556)	-22.92 (7.607)	0.0045
	95% CI for LS MEAN	(-30.25, -13.46)	(-12.18, 14.31)	(-38.29, -7.54)	
Week 8	LS Mean (SEM)	-21.06 (4.147)	-16.21 (6.329)	-4.85 (7.386)	0.5156
	95% CI for LS MEAN	(-29.43, -12.69)	(-29.02, -3.40)	(-19.81, 10.11)	
Week 12	LS Mean (SEM)	-21.30 (3.998)	-15.60 (6.072)	-5.70 (7.103)	0.4276
	95% CI for LS MEAN	(-29.39, -13.22)	(-27.91, -3.30)	(-20.10, 8.70)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:08

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea

Subgroup by treatment interaction p-value	0.1885
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia
Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-6.30 (5.644)	3.57 (8.206)	-9.87 (9.873)	0.3248
	95% CI for LS MEAN	(-17.81, 5.20)	(-13.14, 20.29)	(-30.00, 10.25)	
Week 4	LS Mean (SEM)	-1.29 (6.056)	3.57 (8.896)	-4.86 (10.678)	0.6521
	95% CI for LS MEAN	(-13.60, 11.03)	(-14.50, 21.64)	(-26.56, 16.85)	
Week 8	LS Mean (SEM)	-8.02 (5.305)	-11.65 (8.789)	3.63 (10.130)	0.7240
	95% CI for LS MEAN	(-19.14, 3.10)	(-29.89, 6.59)	(-17.50, 24.75)	
Week 12	LS Mean (SEM)	-7.58 (5.629)	6.35 (8.053)	-13.93 (9.730)	0.1621
	95% CI for LS MEAN	(-19.04, 3.88)	(-10.05, 22.75)	(-33.76, 5.90)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia
Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-3.00 (3.748)	-1.92 (6.017)	-1.08 (6.942)	0.8776
	95% CI for LS MEAN	(-10.59, 4.59)	(-14.10, 10.26)	(-15.15, 13.00)	
Week 4	LS Mean (SEM)	-8.23 (4.124)	-8.54 (6.535)	0.31 (7.607)	0.9673
	95% CI for LS MEAN	(-16.56, 0.10)	(-21.77, 4.68)	(-15.09, 15.71)	
Week 8	LS Mean (SEM)	-13.76 (3.859)	-2.24 (5.840)	-11.52 (6.838)	0.1010
	95% CI for LS MEAN	(-21.57, -5.96)	(-14.09, 9.61)	(-25.41, 2.37)	
Week 12	LS Mean (SEM)	-9.89 (3.623)	-0.21 (5.505)	-9.69 (6.451)	0.1412
	95% CI for LS MEAN	(-17.20, -2.59)	(-11.34, 10.93)	(-22.73, 3.36)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia

Subgroup by treatment interaction p-value 0.9151

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss
Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	3.92 (3.955)	6.67 (5.800)	-2.75 (6.997)	0.6973
	95% CI for LS MEAN	(-4.15, 11.99)	(-5.15, 18.49)	(-17.01, 11.52)	
Week 4	LS Mean (SEM)	-4.08 (3.562)	-1.66 (5.242)	-2.41 (6.312)	0.7045
	95% CI for LS MEAN	(-11.32, 3.17)	(-12.32, 8.99)	(-15.25, 10.42)	
Week 8	LS Mean (SEM)	2.43 (5.555)	12.27 (8.304)	-9.84 (10.033)	0.3365
	95% CI for LS MEAN	(-9.07, 13.93)	(-4.84, 29.38)	(-30.54, 10.86)	
Week 12	LS Mean (SEM)	2.42 (5.613)	6.67 (8.027)	-4.25 (9.778)	0.6665
	95% CI for LS MEAN	(-8.98, 13.82)	(-9.64, 22.98)	(-24.12, 15.62)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss
Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-4.29 (2.662)	2.19 (4.373)	-6.48 (5.041)	0.2055
	95% CI for LS MEAN	(-9.67, 1.08)	(-6.63, 11.01)	(-16.67, 3.70)	
Week 4	LS Mean (SEM)	-5.47 (2.874)	-1.11 (4.571)	-4.36 (5.341)	0.4195
	95% CI for LS MEAN	(-11.26, 0.33)	(-10.34, 8.12)	(-15.14, 6.43)	
Week 8	LS Mean (SEM)	2.46 (3.783)	-0.97 (5.801)	3.43 (6.865)	0.6204
	95% CI for LS MEAN	(-5.18, 10.10)	(-12.70, 10.76)	(-10.46, 17.32)	
Week 12	LS Mean (SEM)	-6.78 (2.955)	-1.11 (4.543)	-5.66 (5.366)	0.2982
	95% CI for LS MEAN	(-12.75, -0.80)	(-10.33, 8.11)	(-16.55, 5.22)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss

Subgroup by treatment interaction p-value	0.8330
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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation
Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.88 (4.488)	3.00 (6.516)	-4.89 (7.836)	0.5377
	95% CI for LS MEAN	(-11.08, 7.31)	(-10.31, 16.32)	(-20.93, 11.16)	
Week 4	LS Mean (SEM)	-6.15 (3.831)	11.34 (5.660)	-17.49 (6.759)	0.0143
	95% CI for LS MEAN	(-13.95, 1.65)	(-0.17, 22.84)	(-31.24, -3.73)	
Week 8	LS Mean (SEM)	-4.39 (6.252)	21.25 (9.247)	-25.64 (11.071)	0.0442
	95% CI for LS MEAN	(-18.47, 9.69)	(0.68, 41.82)	(-50.46, -0.82)	
Week 12	LS Mean (SEM)	-5.61 (4.476)	8.56 (6.498)	-14.17 (7.824)	0.0818
	95% CI for LS MEAN	(-14.82, 3.59)	(-4.77, 21.89)	(-30.27, 1.92)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation
Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.42 (3.599)	11.99 (5.743)	-14.41 (6.620)	0.0355
	95% CI for LS MEAN	(-9.69, 4.85)	(0.40, 23.59)	(-27.79, -1.03)	
Week 4	LS Mean (SEM)	1.16 (4.071)	8.65 (6.461)	-7.49 (7.509)	0.3244
	95% CI for LS MEAN	(-7.05, 9.36)	(-4.39, 21.69)	(-22.65, 7.67)	
Week 8	LS Mean (SEM)	-5.90 (3.791)	6.57 (5.729)	-12.46 (6.682)	0.0702
	95% CI for LS MEAN	(-13.56, 1.76)	(-5.03, 18.17)	(-26.01, 1.08)	
Week 12	LS Mean (SEM)	1.22 (3.816)	8.65 (5.850)	-7.43 (6.845)	0.2848
	95% CI for LS MEAN	(-6.49, 8.93)	(-3.20, 20.49)	(-21.28, 6.43)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation

Subgroup by treatment interaction p-value	0.8540
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea
Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.15 (3.740)	-8.44 (5.414)	6.29 (6.658)	0.3508
	95% CI for LS MEAN	(-9.74, 5.44)	(-19.43, 2.54)	(-7.20, 19.79)	
Week 4	LS Mean (SEM)	-2.23 (2.910)	-8.44 (4.300)	6.21 (5.286)	0.2475
	95% CI for LS MEAN	(-8.14, 3.67)	(-17.16, 0.27)	(-4.50, 16.92)	
Week 8	LS Mean (SEM)	-1.04 (3.621)	1.27 (6.199)	-2.31 (7.321)	0.7547
	95% CI for LS MEAN	(-8.55, 6.47)	(-11.49, 14.03)	(-17.36, 12.74)	
Week 12	LS Mean (SEM)	-3.26 (3.294)	-5.67 (4.638)	2.40 (5.768)	0.6796
	95% CI for LS MEAN	(-9.99, 3.46)	(-15.12, 3.79)	(-9.35, 14.15)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:08

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea
Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.21 (2.597)	3.47 (4.360)	-3.68 (4.993)	0.4661
	95% CI for LS MEAN	(-5.47, 5.05)	(-5.37, 12.31)	(-13.82, 6.46)	
Week 4	LS Mean (SEM)	-1.25 (2.277)	-0.07 (3.608)	-1.19 (4.200)	0.7791
	95% CI for LS MEAN	(-5.85, 3.35)	(-7.36, 7.23)	(-9.68, 7.31)	
Week 8	LS Mean (SEM)	1.91 (2.827)	-2.95 (4.425)	4.86 (5.200)	0.3555
	95% CI for LS MEAN	(-3.79, 7.61)	(-11.89, 6.00)	(-5.65, 15.38)	
Week 12	LS Mean (SEM)	1.48 (2.487)	-2.84 (3.885)	4.32 (4.553)	0.3499
	95% CI for LS MEAN	(-3.58, 6.54)	(-10.78, 5.09)	(-4.97, 13.62)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea

Subgroup by treatment interaction p-value	0.5502
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties
Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.94 (4.707)	6.43 (6.843)	-7.37 (8.310)	0.3813
	95% CI for LS MEAN	(-10.50, 8.62)	(-7.46, 20.32)	(-24.24, 9.50)	
Week 4	LS Mean (SEM)	1.72 (5.318)	3.65 (7.720)	-1.92 (9.379)	0.8387
	95% CI for LS MEAN	(-9.07, 12.52)	(-12.02, 19.32)	(-20.96, 17.11)	
Week 8	LS Mean (SEM)	-6.51 (2.801)	-11.49 (4.211)	4.98 (4.977)	0.3337
	95% CI for LS MEAN	(-12.51, -0.52)	(-20.44, -2.54)	(-5.66, 15.61)	
Week 12	LS Mean (SEM)	-0.56 (4.306)	6.43 (6.256)	-6.99 (7.602)	0.3641
	95% CI for LS MEAN	(-9.31, 8.18)	(-6.27, 19.13)	(-22.42, 8.44)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties
Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.05 (2.874)	0.78 (4.707)	-2.83 (5.387)	0.6020
	95% CI for LS MEAN	(-7.87, 3.76)	(-8.74, 10.30)	(-13.74, 8.08)	
Week 4	LS Mean (SEM)	-5.18 (2.707)	-9.30 (4.230)	4.12 (4.913)	0.4069
	95% CI for LS MEAN	(-10.65, 0.29)	(-17.86, -0.74)	(-5.82, 14.06)	
Week 8	LS Mean (SEM)	-6.58 (4.118)	-0.16 (6.351)	-6.42 (7.491)	0.3965
	95% CI for LS MEAN	(-14.89, 1.73)	(-13.01, 12.70)	(-21.59, 8.74)	
Week 12	LS Mean (SEM)	-3.32 (3.820)	-3.74 (5.949)	0.42 (6.992)	0.9528
	95% CI for LS MEAN	(-11.02, 4.37)	(-15.76, 8.28)	(-13.71, 14.54)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.4
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties

Subgroup by treatment interaction p-value	0.6700
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.5
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue
> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-12.50 (4.125)	-7.50 (6.747)	-5.00 (7.493)	0.5099
	95% CI for LS MEAN	(-20.90, -4.09)	(-21.22, 6.22)	(-20.29, 10.30)	
Week 4	LS Mean (SEM)	-18.05 (4.075)	6.97 (6.362)	-25.02 (7.170)	0.0015
	95% CI for LS MEAN	(-26.35, -9.75)	(-6.00, 19.94)	(-39.66, -10.39)	
Week 8	LS Mean (SEM)	-12.27 (5.415)	-10.34 (9.275)	-1.94 (10.455)	0.8547
	95% CI for LS MEAN	(-23.38, -1.17)	(-29.40, 8.73)	(-23.49, 19.62)	
Week 12	LS Mean (SEM)	-17.36 (3.817)	-12.78 (5.804)	-4.58 (6.544)	0.4895
	95% CI for LS MEAN	(-25.15, -9.57)	(-24.64, -0.92)	(-17.96, 8.80)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.5
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue
≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-11.10 (3.494)	4.65 (5.091)	-15.74 (6.175)	0.0145
	95% CI for LS MEAN	(-18.15, -4.05)	(-5.63, 14.92)	(-28.21, -3.28)	
Week 4	LS Mean (SEM)	-14.75 (3.395)	-2.02 (5.037)	-12.73 (6.074)	0.0418
	95% CI for LS MEAN	(-21.58, -7.91)	(-12.16, 8.12)	(-24.96, -0.50)	
Week 8	LS Mean (SEM)	-15.81 (3.234)	1.87 (4.749)	-17.68 (5.738)	0.0042
	95% CI for LS MEAN	(-22.41, -9.22)	(-7.80, 11.54)	(-29.38, -5.99)	
Week 12	LS Mean (SEM)	-15.33 (3.831)	6.13 (5.553)	-21.46 (6.745)	0.0027
	95% CI for LS MEAN	(-23.04, -7.62)	(-5.06, 17.32)	(-35.05, -7.87)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.5

Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue

Subgroup by treatment interaction p-value	0.6736
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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.5
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting
> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	4.43 (2.481)	1.64 (4.115)	2.79 (4.750)	0.5612
	95% CI for LS MEAN	(-0.62, 9.47)	(-6.72, 9.99)	(-6.87, 12.45)	
Week 4	LS Mean (SEM)	3.73 (1.894)	1.48 (2.988)	2.25 (3.466)	0.5215
	95% CI for LS MEAN	(-0.13, 7.59)	(-4.61, 7.58)	(-4.82, 9.32)	
Week 8	LS Mean (SEM)	7.72 (4.315)	-1.10 (7.133)	8.81 (8.262)	0.2972
	95% CI for LS MEAN	(-1.20, 16.63)	(-15.83, 13.64)	(-8.27, 25.90)	
Week 12	LS Mean (SEM)	5.64 (2.785)	-2.22 (4.433)	7.86 (5.184)	0.1445
	95% CI for LS MEAN	(-0.13, 11.40)	(-11.43, 6.99)	(-2.92, 18.64)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.5
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting
≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.44 (1.760)	0.30 (2.551)	-1.74 (3.103)	0.5783
	95% CI for LS MEAN	(-4.99, 2.11)	(-4.85, 5.44)	(-7.99, 4.52)	
Week 4	LS Mean (SEM)	-0.73 (1.797)	-4.15 (2.667)	3.42 (3.219)	0.2941
	95% CI for LS MEAN	(-4.36, 2.90)	(-9.53, 1.24)	(-3.08, 9.92)	
Week 8	LS Mean (SEM)	-1.16 (2.110)	0.06 (3.119)	-1.22 (3.763)	0.7477
	95% CI for LS MEAN	(-5.45, 3.12)	(-6.28, 6.40)	(-8.87, 6.43)	
Week 12	LS Mean (SEM)	-1.25 (2.421)	2.52 (3.417)	-3.77 (4.190)	0.3737
	95% CI for LS MEAN	(-6.13, 3.63)	(-4.37, 9.41)	(-12.21, 4.68)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.5

Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting

Subgroup by treatment interaction p-value	0.8837
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.5
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain
> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	2.12 (3.491)	4.54 (5.918)	-2.42 (6.621)	0.7177
	95% CI for LS MEAN	(-4.98, 9.22)	(-7.48, 16.55)	(-15.90, 11.07)	
Week 4	LS Mean (SEM)	1.43 (2.710)	-1.54 (4.284)	2.97 (4.777)	0.5392
	95% CI for LS MEAN	(-4.10, 6.95)	(-10.28, 7.20)	(-6.79, 12.73)	
Week 8	LS Mean (SEM)	6.27 (5.717)	-3.25 (9.869)	9.52 (11.283)	0.4065
	95% CI for LS MEAN	(-5.45, 17.98)	(-23.51, 17.01)	(-13.67, 32.71)	
Week 12	LS Mean (SEM)	1.11 (3.524)	2.16 (5.518)	-1.05 (6.323)	0.8693
	95% CI for LS MEAN	(-6.14, 8.36)	(-9.23, 13.56)	(-14.15, 12.04)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain
≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-5.99 (2.605)	2.43 (3.888)	-8.42 (4.680)	0.0788
	95% CI for LS MEAN	(-11.24, -0.74)	(-5.40, 10.27)	(-17.85, 1.01)	
Week 4	LS Mean (SEM)	-4.41 (3.211)	4.13 (4.764)	-8.54 (5.745)	0.1443
	95% CI for LS MEAN	(-10.89, 2.06)	(-5.47, 13.73)	(-20.13, 3.04)	
Week 8	LS Mean (SEM)	-4.64 (4.133)	-5.36 (6.232)	0.72 (7.479)	0.9236
	95% CI for LS MEAN	(-13.06, 3.79)	(-18.06, 7.34)	(-14.52, 15.97)	
Week 12	LS Mean (SEM)	-4.71 (4.277)	6.49 (6.317)	-11.20 (7.629)	0.1491
	95% CI for LS MEAN	(-13.33, 3.91)	(-6.24, 19.23)	(-26.58, 4.17)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain

Subgroup by treatment interaction p-value	0.2984
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.5
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea
> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-14.43 (4.892)	-13.91 (7.957)	-0.51 (8.851)	0.9543
	95% CI for LS MEAN	(-24.40, -4.45)	(-30.12, 2.29)	(-18.61, 17.59)	
Week 4	LS Mean (SEM)	-19.98 (5.023)	-10.03 (7.926)	-9.95 (8.947)	0.2747
	95% CI for LS MEAN	(-30.20, -9.77)	(-26.18, 6.12)	(-28.21, 8.31)	
Week 8	LS Mean (SEM)	-11.75 (4.929)	-18.23 (8.231)	6.48 (9.101)	0.4831
	95% CI for LS MEAN	(-21.84, -1.66)	(-35.10, -1.37)	(-12.27, 25.22)	
Week 12	LS Mean (SEM)	-20.17 (4.350)	-6.33 (6.660)	-13.84 (7.461)	0.0753
	95% CI for LS MEAN	(-29.09, -11.24)	(-20.01, 7.36)	(-29.20, 1.52)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.5
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea
≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-15.18 (3.550)	8.76 (5.213)	-23.94 (6.321)	0.0005
	95% CI for LS MEAN	(-22.35, -8.01)	(-1.77, 19.29)	(-36.71, -11.18)	
Week 4	LS Mean (SEM)	-24.41 (4.018)	6.54 (5.975)	-30.95 (7.208)	<0.0001
	95% CI for LS MEAN	(-32.51, -16.32)	(-5.50, 18.57)	(-45.47, -16.43)	
Week 8	LS Mean (SEM)	-23.25 (4.264)	1.87 (6.296)	-25.12 (7.610)	0.0023
	95% CI for LS MEAN	(-31.91, -14.59)	(-10.93, 14.66)	(-40.59, -9.65)	
Week 12	LS Mean (SEM)	-19.03 (4.394)	-4.57 (6.326)	-14.46 (7.708)	0.0678
	95% CI for LS MEAN	(-27.90, -10.17)	(-17.35, 8.20)	(-30.02, 1.11)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.5

Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea

Subgroup by treatment interaction p-value	0.0726
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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia
> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	0.70 (5.676)	-2.18 (9.313)	2.88 (10.443)	0.7846
	95% CI for LS MEAN	(-10.90, 12.30)	(-21.19, 16.82)	(-18.53, 24.30)	
Week 4	LS Mean (SEM)	0.70 (5.296)	4.97 (8.299)	-4.27 (9.365)	0.6521
	95% CI for LS MEAN	(-10.11, 11.50)	(-12.00, 21.94)	(-23.47, 14.93)	
Week 8	LS Mean (SEM)	-2.54 (5.104)	-7.69 (8.596)	5.15 (9.373)	0.5900
	95% CI for LS MEAN	(-13.25, 8.18)	(-25.77, 10.38)	(-14.73, 25.04)	
Week 12	LS Mean (SEM)	-3.01 (5.202)	1.26 (7.969)	-4.28 (9.027)	0.6394
	95% CI for LS MEAN	(-13.61, 7.59)	(-15.02, 17.54)	(-22.74, 14.19)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.5
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia
≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-7.92 (3.783)	1.19 (5.497)	-9.10 (6.674)	0.1798
	95% CI for LS MEAN	(-15.55, -0.29)	(-9.91, 12.28)	(-22.57, 4.36)	
Week 4	LS Mean (SEM)	-9.39 (4.741)	-7.70 (7.037)	-1.69 (8.485)	0.8429
	95% CI for LS MEAN	(-18.94, 0.15)	(-21.87, 6.47)	(-18.78, 15.39)	
Week 8	LS Mean (SEM)	-17.50 (4.235)	-6.78 (6.229)	-10.73 (7.527)	0.1628
	95% CI for LS MEAN	(-26.09, -8.92)	(-19.41, 5.85)	(-25.99, 4.54)	
Week 12	LS Mean (SEM)	-13.63 (3.993)	3.41 (5.697)	-17.04 (6.955)	0.0183
	95% CI for LS MEAN	(-21.68, -5.59)	(-8.08, 14.90)	(-31.06, -3.03)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.5

Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia

Subgroup by treatment interaction p-value	0.2948
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.5
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss
> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	2.10 (4.254)	0.50 (7.061)	1.60 (7.982)	0.8422
	95% CI for LS MEAN	(-6.55, 10.76)	(-13.85, 14.85)	(-14.67, 17.87)	
Week 4	LS Mean (SEM)	-4.84 (3.239)	-3.31 (4.996)	-1.53 (5.650)	0.7881
	95% CI for LS MEAN	(-11.44, 1.76)	(-13.50, 6.88)	(-13.07, 10.01)	
Week 8	LS Mean (SEM)	1.93 (3.917)	-1.27 (6.545)	3.21 (7.335)	0.6659
	95% CI for LS MEAN	(-6.13, 10.00)	(-14.73, 12.18)	(-11.94, 18.36)	
Week 12	LS Mean (SEM)	1.00 (5.710)	0.39 (8.931)	0.60 (10.425)	0.9542
	95% CI for LS MEAN	(-10.67, 12.67)	(-17.93, 18.72)	(-20.81, 22.02)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.5
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss
≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.30 (2.487)	5.05 (3.694)	-7.36 (4.494)	0.1088
	95% CI for LS MEAN	(-7.32, 2.71)	(-2.39, 12.50)	(-16.42, 1.70)	
Week 4	LS Mean (SEM)	-4.34 (3.111)	-1.61 (4.642)	-2.73 (5.622)	0.6301
	95% CI for LS MEAN	(-10.61, 1.93)	(-10.97, 7.74)	(-14.05, 8.60)	
Week 8	LS Mean (SEM)	2.07 (4.215)	4.78 (6.227)	-2.70 (7.547)	0.7221
	95% CI for LS MEAN	(-6.45, 10.60)	(-7.82, 17.37)	(-17.97, 12.56)	
Week 12	LS Mean (SEM)	-4.25 (3.473)	2.83 (5.060)	-7.08 (6.169)	0.2569
	95% CI for LS MEAN	(-11.24, 2.74)	(-7.36, 13.03)	(-19.51, 5.34)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.5

Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss

Subgroup by treatment interaction p-value	0.6242
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.5
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation
> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.39 (4.978)	4.22 (8.138)	-5.61 (9.143)	0.5441
	95% CI for LS MEAN	(-11.53, 8.74)	(-12.33, 20.76)	(-24.27, 13.05)	
Week 4	LS Mean (SEM)	-0.01 (5.053)	8.38 (8.001)	-8.38 (9.099)	0.3640
	95% CI for LS MEAN	(-10.28, 10.27)	(-7.92, 24.67)	(-26.95, 10.18)	
Week 8	LS Mean (SEM)	-4.07 (4.194)	12.92 (6.728)	-16.99 (7.359)	0.0284
	95% CI for LS MEAN	(-12.62, 4.48)	(-0.79, 26.64)	(-32.06, -1.93)	
Week 12	LS Mean (SEM)	1.55 (5.507)	15.79 (8.655)	-14.23 (9.909)	0.1612
	95% CI for LS MEAN	(-9.64, 12.75)	(-1.86, 33.43)	(-34.47, 6.00)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.5
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation
≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-3.80 (3.245)	6.29 (4.757)	-10.09 (5.760)	0.0868
	95% CI for LS MEAN	(-10.35, 2.74)	(-3.31, 15.88)	(-21.71, 1.52)	
Week 4	LS Mean (SEM)	-4.64 (3.328)	8.51 (4.938)	-13.15 (5.956)	0.0324
	95% CI for LS MEAN	(-11.35, 2.06)	(-1.44, 18.46)	(-25.15, -1.15)	
Week 8	LS Mean (SEM)	-8.21 (3.564)	5.87 (5.254)	-14.09 (6.352)	0.0355
	95% CI for LS MEAN	(-15.54, -0.89)	(-4.93, 16.68)	(-27.15, -1.03)	
Week 12	LS Mean (SEM)	-5.43 (2.860)	1.84 (4.087)	-7.27 (4.987)	0.1520
	95% CI for LS MEAN	(-11.20, 0.34)	(-6.40, 10.09)	(-17.34, 2.79)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.5
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation

Subgroup by treatment interaction p-value	0.9985
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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea
> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	0.72 (1.885)	0.44 (3.092)	0.28 (3.361)	0.9331
	95% CI for LS MEAN	(-3.13, 4.57)	(-5.86, 6.73)	(-6.58, 7.15)	
Week 4	LS Mean (SEM)	2.11 (2.476)	0.06 (3.916)	2.05 (4.465)	0.6491
	95% CI for LS MEAN	(-2.93, 7.15)	(-7.94, 8.05)	(-7.07, 11.18)	
Week 8	LS Mean (SEM)	8.70 (3.841)	-0.09 (6.446)	8.79 (7.407)	0.2461
	95% CI for LS MEAN	(0.82, 16.57)	(-13.30, 13.12)	(-6.42, 24.00)	
Week 12	LS Mean (SEM)	8.26 (3.216)	0.06 (5.061)	8.21 (5.869)	0.1753
	95% CI for LS MEAN	(1.65, 14.87)	(-10.39, 10.50)	(-3.93, 20.34)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.5
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea
≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.87 (3.453)	-4.91 (5.071)	4.03 (6.143)	0.5146
	95% CI for LS MEAN	(-7.82, 6.08)	(-15.12, 5.30)	(-8.33, 16.40)	
Week 4	LS Mean (SEM)	-3.08 (2.533)	-7.13 (3.765)	4.04 (4.549)	0.3787
	95% CI for LS MEAN	(-8.19, 2.02)	(-14.71, 0.46)	(-5.12, 13.21)	
Week 8	LS Mean (SEM)	-2.45 (2.592)	-3.53 (3.833)	1.08 (4.641)	0.8176
	95% CI for LS MEAN	(-7.70, 2.80)	(-11.29, 4.23)	(-8.32, 10.47)	
Week 12	LS Mean (SEM)	-6.04 (2.417)	-7.13 (3.411)	1.09 (4.188)	0.7957
	95% CI for LS MEAN	(-10.92, -1.15)	(-14.02, -0.24)	(-7.37, 9.55)	

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Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.5

Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea

Subgroup by treatment interaction p-value	0.7147
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.5
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties
> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.65 (4.119)	-8.06 (6.799)	7.41 (7.601)	0.3374
	95% CI for LS MEAN	(-9.05, 7.74)	(-21.90, 5.77)	(-8.11, 22.94)	
Week 4	LS Mean (SEM)	-2.04 (4.283)	-12.21 (6.886)	10.17 (7.806)	0.2028
	95% CI for LS MEAN	(-10.77, 6.69)	(-26.26, 1.84)	(-5.79, 26.14)	
Week 8	LS Mean (SEM)	-7.82 (3.917)	-4.96 (6.525)	-2.86 (7.250)	0.6962
	95% CI for LS MEAN	(-15.86, 0.21)	(-18.36, 8.44)	(-17.81, 12.08)	
Week 12	LS Mean (SEM)	-1.51 (4.576)	-4.80 (7.297)	3.30 (8.319)	0.6946
	95% CI for LS MEAN	(-10.81, 7.79)	(-19.66, 10.05)	(-13.67, 20.27)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.5
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties
≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-3.47 (3.281)	8.88 (4.867)	-12.35 (5.870)	0.0412
	95% CI for LS MEAN	(-10.09, 3.15)	(-0.93, 18.70)	(-24.19, -0.52)	
Week 4	LS Mean (SEM)	-3.47 (3.510)	2.21 (5.206)	-5.69 (6.279)	0.3702
	95% CI for LS MEAN	(-10.55, 3.61)	(-8.29, 12.72)	(-18.35, 6.98)	
Week 8	LS Mean (SEM)	-5.79 (4.287)	6.87 (6.319)	-12.66 (7.636)	0.1096
	95% CI for LS MEAN	(-14.60, 3.03)	(-6.13, 19.87)	(-28.36, 3.05)	
Week 12	LS Mean (SEM)	-3.84 (3.629)	4.44 (5.272)	-8.27 (6.400)	0.2028
	95% CI for LS MEAN	(-11.14, 3.47)	(-6.19, 15.07)	(-21.17, 4.62)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.5

Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties

Subgroup by treatment interaction p-value	0.0685
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue
Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-13.52 (2.963)	-3.85 (4.447)	-9.67 (5.347)	0.0770
	95% CI for LS MEAN	(-19.48, -7.56)	(-12.80, 5.10)	(-20.43, 1.09)	
Week 4	LS Mean (SEM)	-17.13 (3.069)	3.56 (4.691)	-20.69 (5.608)	0.0006
	95% CI for LS MEAN	(-23.31, -10.95)	(-5.88, 13.00)	(-31.97, -9.40)	
Week 8	LS Mean (SEM)	-9.81 (3.551)	-5.05 (5.463)	-4.76 (6.518)	0.4690
	95% CI for LS MEAN	(-16.96, -2.66)	(-16.05, 5.94)	(-17.88, 8.36)	
Week 12	LS Mean (SEM)	-14.07 (3.692)	-2.37 (5.558)	-11.70 (6.674)	0.0867
	95% CI for LS MEAN	(-21.51, -6.63)	(-13.58, 8.84)	(-25.16, 1.76)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue
Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-9.96 (5.235)	9.84 (8.388)	-19.80 (9.732)	0.0523
	95% CI for LS MEAN	(-20.73, 0.80)	(-7.36, 27.04)	(-39.82, 0.21)	
Week 4	LS Mean (SEM)	-15.52 (4.803)	-0.84 (7.445)	-14.68 (8.684)	0.1020
	95% CI for LS MEAN	(-25.33, -5.70)	(-16.06, 14.39)	(-32.47, 3.11)	
Week 8	LS Mean (SEM)	-26.13 (3.354)	-4.33 (5.394)	-21.79 (5.994)	0.0025
	95% CI for LS MEAN	(-33.21, -19.05)	(-15.76, 7.09)	(-34.58, -9.01)	
Week 12	LS Mean (SEM)	-18.46 (4.493)	2.87 (6.749)	-21.33 (7.932)	0.0121
	95% CI for LS MEAN	(-27.65, -9.27)	(-10.96, 16.69)	(-37.60, -5.06)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue

Subgroup by treatment interaction p-value 0.2221

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting
Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	0.52 (1.826)	2.40 (2.715)	-1.88 (3.282)	0.5693
	95% CI for LS MEAN	(-3.17, 4.20)	(-3.08, 7.89)	(-8.51, 4.74)	
Week 4	LS Mean (SEM)	0.71 (1.588)	-0.93 (2.432)	1.64 (2.916)	0.5771
	95% CI for LS MEAN	(-2.49, 3.90)	(-5.83, 3.96)	(-4.23, 7.50)	
Week 8	LS Mean (SEM)	2.24 (2.856)	-0.34 (4.451)	2.59 (5.295)	0.6279
	95% CI for LS MEAN	(-3.53, 8.02)	(-9.34, 8.66)	(-8.12, 13.30)	
Week 12	LS Mean (SEM)	0.23 (1.486)	-2.04 (2.165)	2.28 (2.638)	0.3943
	95% CI for LS MEAN	(-2.79, 3.25)	(-6.45, 2.37)	(-3.09, 7.65)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting
Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.29 (2.510)	-0.65 (3.989)	0.36 (4.543)	0.9368
	95% CI for LS MEAN	(-5.42, 4.85)	(-8.78, 7.49)	(-8.94, 9.66)	
Week 4	LS Mean (SEM)	-0.29 (2.323)	-2.20 (3.539)	1.91 (4.056)	0.6414
	95% CI for LS MEAN	(-5.06, 4.49)	(-9.48, 5.09)	(-6.46, 10.29)	
Week 8	LS Mean (SEM)	-1.68 (2.225)	6.41 (3.549)	-8.09 (3.979)	0.0522
	95% CI for LS MEAN	(-6.24, 2.87)	(-0.85, 13.67)	(-16.27, 0.08)	
Week 12	LS Mean (SEM)	-0.86 (3.199)	7.06 (4.805)	-7.92 (5.643)	0.1714
	95% CI for LS MEAN	(-7.39, 5.67)	(-2.77, 16.89)	(-19.48, 3.64)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting

Subgroup by treatment interaction p-value	0.3696
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain
Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.34 (2.788)	2.22 (4.322)	-4.56 (5.165)	0.3817
	95% CI for LS MEAN	(-7.96, 3.27)	(-6.48, 10.92)	(-14.96, 5.84)	
Week 4	LS Mean (SEM)	-4.27 (2.449)	-1.48 (3.779)	-2.79 (4.534)	0.5409
	95% CI for LS MEAN	(-9.20, 0.65)	(-9.08, 6.12)	(-11.91, 6.32)	
Week 8	LS Mean (SEM)	1.76 (4.881)	-9.25 (7.783)	11.01 (9.209)	0.2397
	95% CI for LS MEAN	(-8.14, 11.65)	(-25.03, 6.53)	(-7.66, 29.68)	
Week 12	LS Mean (SEM)	-5.36 (2.872)	-3.25 (4.398)	-2.11 (5.279)	0.6912
	95% CI for LS MEAN	(-11.15, 0.44)	(-12.12, 5.63)	(-12.76, 8.54)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain
Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.71 (3.587)	8.37 (5.612)	-11.07 (6.200)	0.0849
	95% CI for LS MEAN	(-10.05, 4.63)	(-3.09, 19.82)	(-23.77, 1.62)	
Week 4	LS Mean (SEM)	1.84 (4.412)	11.95 (6.735)	-10.11 (7.738)	0.2025
	95% CI for LS MEAN	(-7.18, 10.86)	(-1.84, 25.73)	(-25.98, 5.76)	
Week 8	LS Mean (SEM)	-2.49 (4.065)	4.82 (6.470)	-7.31 (7.255)	0.3263
	95% CI for LS MEAN	(-10.94, 5.96)	(-8.59, 18.23)	(-22.48, 7.86)	
Week 12	LS Mean (SEM)	2.63 (5.652)	21.20 (8.574)	-18.57 (10.011)	0.0752
	95% CI for LS MEAN	(-8.94, 14.21)	(3.60, 38.81)	(-39.17, 2.02)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain

Subgroup by treatment interaction p-value	0.2331
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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

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by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea
Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-13.03 (3.717)	-6.80 (5.633)	-6.23 (6.763)	0.3623
	95% CI for LS MEAN	(-20.52, -5.54)	(-18.17, 4.56)	(-19.87, 7.41)	
Week 4	LS Mean (SEM)	-18.92 (4.131)	-2.36 (6.317)	-16.56 (7.557)	0.0334
	95% CI for LS MEAN	(-27.23, -10.61)	(-15.06, 10.35)	(-31.76, -1.36)	
Week 8	LS Mean (SEM)	-12.36 (4.223)	-7.07 (6.516)	-5.28 (7.774)	0.5003
	95% CI for LS MEAN	(-20.87, -3.85)	(-20.20, 6.05)	(-20.94, 10.38)	
Week 12	LS Mean (SEM)	-16.93 (3.735)	-4.58 (5.580)	-12.34 (6.724)	0.0736
	95% CI for LS MEAN	(-24.46, -9.39)	(-15.85, 6.69)	(-25.92, 1.23)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea
Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-14.62 (4.408)	17.34 (7.078)	-31.97 (7.912)	0.0005
	95% CI for LS MEAN	(-23.69, -5.56)	(2.84, 31.85)	(-48.28, -15.66)	
Week 4	LS Mean (SEM)	-25.23 (4.665)	7.51 (7.170)	-32.74 (8.182)	0.0004
	95% CI for LS MEAN	(-34.76, -15.70)	(-7.16, 22.18)	(-49.52, -15.96)	
Week 8	LS Mean (SEM)	-28.52 (4.175)	-2.63 (6.535)	-25.89 (7.301)	0.0035
	95% CI for LS MEAN	(-37.42, -19.63)	(-16.56, 11.30)	(-41.63, -10.15)	
Week 12	LS Mean (SEM)	-20.08 (5.403)	-3.60 (8.060)	-16.48 (9.395)	0.0913
	95% CI for LS MEAN	(-31.15, -9.01)	(-20.14, 12.94)	(-35.80, 2.84)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea

Subgroup by treatment interaction p-value	0.0628
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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia
Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-5.73 (3.642)	-1.53 (5.458)	-4.19 (6.565)	0.5263
	95% CI for LS MEAN	(-13.05, 1.60)	(-12.52, 9.46)	(-17.41, 9.02)	
Week 4	LS Mean (SEM)	-5.99 (3.802)	0.69 (5.810)	-6.68 (6.947)	0.3417
	95% CI for LS MEAN	(-13.64, 1.67)	(-11.01, 12.39)	(-20.66, 7.31)	
Week 8	LS Mean (SEM)	-10.10 (3.415)	-2.96 (5.271)	-7.15 (6.283)	0.2624
	95% CI for LS MEAN	(-17.02, -3.19)	(-13.62, 7.71)	(-19.86, 5.57)	
Week 12	LS Mean (SEM)	-9.05 (4.161)	7.36 (6.174)	-16.40 (7.447)	0.0331
	95% CI for LS MEAN	(-17.43, -0.66)	(-5.10, 19.81)	(-31.42, -1.38)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia
Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.51 (5.751)	4.50 (9.168)	-7.01 (10.271)	0.5005
	95% CI for LS MEAN	(-14.28, 9.26)	(-14.21, 23.21)	(-28.07, 14.05)	
Week 4	LS Mean (SEM)	-4.03 (7.044)	-8.99 (10.803)	4.96 (12.493)	0.6943
	95% CI for LS MEAN	(-18.41, 10.35)	(-31.08, 13.10)	(-20.64, 30.56)	
Week 8	LS Mean (SEM)	-13.81 (7.947)	-3.74 (12.631)	-10.07 (14.579)	0.5004
	95% CI for LS MEAN	(-30.63, 3.01)	(-30.46, 22.97)	(-41.13, 20.99)	
Week 12	LS Mean (SEM)	-8.61 (4.928)	-5.29 (7.243)	-3.33 (8.194)	0.6880
	95% CI for LS MEAN	(-18.71, 1.49)	(-20.15, 9.58)	(-20.15, 13.50)	

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Modified Randomized Set

Insomnia

Subgroup by treatment interaction p-value	0.6909
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Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss
Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	2.28 (3.156)	4.03 (4.790)	-1.75 (5.762)	0.7629
	95% CI for LS MEAN	(-4.07, 8.63)	(-5.61, 13.67)	(-13.34, 9.85)	
Week 4	LS Mean (SEM)	-2.86 (3.024)	-0.42 (4.630)	-2.44 (5.558)	0.6626
	95% CI for LS MEAN	(-8.94, 3.23)	(-9.74, 8.90)	(-13.62, 8.74)	
Week 8	LS Mean (SEM)	4.94 (4.119)	0.07 (6.344)	4.87 (7.587)	0.5258
	95% CI for LS MEAN	(-3.47, 13.36)	(-12.88, 13.02)	(-10.62, 20.36)	
Week 12	LS Mean (SEM)	-0.28 (4.048)	-0.42 (6.068)	0.14 (7.316)	0.9850
	95% CI for LS MEAN	(-8.46, 7.90)	(-12.70, 11.86)	(-14.66, 14.94)	

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Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss
Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-3.95 (2.916)	4.16 (4.733)	-8.11 (5.379)	0.1419
	95% CI for LS MEAN	(-9.91, 2.01)	(-5.47, 13.79)	(-19.09, 2.87)	
Week 4	LS Mean (SEM)	-6.98 (3.123)	-3.59 (4.833)	-3.39 (5.610)	0.5502
	95% CI for LS MEAN	(-13.37, -0.59)	(-13.49, 6.31)	(-14.89, 8.11)	
Week 8	LS Mean (SEM)	-1.61 (4.822)	11.25 (7.753)	-12.86 (9.003)	0.1672
	95% CI for LS MEAN	(-11.59, 8.37)	(-4.78, 27.27)	(-31.52, 5.81)	
Week 12	LS Mean (SEM)	-4.52 (4.580)	7.52 (6.953)	-12.04 (8.220)	0.1537
	95% CI for LS MEAN	(-13.87, 4.82)	(-6.69, 21.74)	(-28.85, 4.77)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss

Subgroup by treatment interaction p-value	0.7097
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation
Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-8.21 (3.380)	4.44 (5.086)	-12.65 (6.128)	0.0446
	95% CI for LS MEAN	(-15.01, -1.41)	(-5.80, 14.68)	(-24.98, -0.32)	
Week 4	LS Mean (SEM)	-7.60 (3.299)	11.10 (5.053)	-18.70 (6.053)	0.0033
	95% CI for LS MEAN	(-14.24, -0.97)	(0.94, 21.27)	(-30.88, -6.53)	
Week 8	LS Mean (SEM)	-8.32 (2.911)	4.43 (4.547)	-12.75 (5.420)	0.0232
	95% CI for LS MEAN	(-14.19, -2.45)	(-4.73, 13.59)	(-23.67, -1.83)	
Week 12	LS Mean (SEM)	-7.16 (3.260)	6.66 (4.821)	-13.82 (5.843)	0.0226
	95% CI for LS MEAN	(-13.73, -0.59)	(-3.07, 16.39)	(-25.60, -2.04)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation
Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.92 (4.004)	5.00 (6.284)	0.92 (6.936)	0.8951
	95% CI for LS MEAN	(-2.28, 14.12)	(-7.82, 17.82)	(-13.27, 15.11)	
Week 4	LS Mean (SEM)	5.92 (4.736)	0.89 (7.232)	5.03 (8.254)	0.5472
	95% CI for LS MEAN	(-3.77, 15.61)	(-13.92, 15.70)	(-11.91, 21.97)	
Week 8	LS Mean (SEM)	-0.97 (5.035)	8.39 (7.751)	-9.36 (8.823)	0.3015
	95% CI for LS MEAN	(-11.43, 9.49)	(-7.74, 24.52)	(-27.76, 9.04)	
Week 12	LS Mean (SEM)	3.72 (5.375)	4.59 (8.123)	-0.87 (9.378)	0.9269
	95% CI for LS MEAN	(-7.37, 14.82)	(-12.23, 21.42)	(-20.37, 18.63)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation

Subgroup by treatment interaction p-value	0.3283
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea
Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.62 (2.251)	-3.18 (3.394)	1.56 (4.087)	0.7047
	95% CI for LS MEAN	(-6.15, 2.92)	(-10.01, 3.66)	(-6.67, 9.79)	
Week 4	LS Mean (SEM)	-0.79 (1.789)	-5.40 (2.742)	4.61 (3.294)	0.1683
	95% CI for LS MEAN	(-4.39, 2.81)	(-10.91, 0.12)	(-2.01, 11.23)	
Week 8	LS Mean (SEM)	2.40 (3.011)	-2.73 (4.682)	5.13 (5.581)	0.3632
	95% CI for LS MEAN	(-3.67, 8.47)	(-12.16, 6.71)	(-6.12, 16.37)	
Week 12	LS Mean (SEM)	0.04 (2.676)	-3.18 (3.905)	3.21 (4.745)	0.5024
	95% CI for LS MEAN	(-5.37, 5.44)	(-11.07, 4.72)	(-6.38, 12.80)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea
Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	1.82 (4.501)	-1.28 (7.129)	3.10 (8.293)	0.7108
	95% CI for LS MEAN	(-7.37, 11.02)	(-15.82, 13.26)	(-13.85, 20.05)	
Week 4	LS Mean (SEM)	-1.21 (3.741)	-1.35 (5.746)	0.14 (6.690)	0.9834
	95% CI for LS MEAN	(-8.85, 6.44)	(-13.11, 10.41)	(-13.58, 13.86)	
Week 8	LS Mean (SEM)	0.06 (2.971)	1.34 (4.677)	-1.28 (5.311)	0.8116
	95% CI for LS MEAN	(-6.09, 6.21)	(-8.33, 11.00)	(-12.32, 9.75)	
Week 12	LS Mean (SEM)	-1.14 (2.514)	-5.05 (3.690)	3.91 (4.222)	0.3623
	95% CI for LS MEAN	(-6.29, 4.02)	(-12.62, 2.52)	(-4.76, 12.58)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea

Subgroup by treatment interaction p-value	0.8053
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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties
Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.90 (2.682)	-2.97 (4.094)	0.07 (4.898)	0.9893
	95% CI for LS MEAN	(-8.31, 2.51)	(-11.23, 5.29)	(-9.82, 9.95)	
Week 4	LS Mean (SEM)	-3.85 (2.745)	-9.63 (4.191)	5.78 (5.014)	0.2552
	95% CI for LS MEAN	(-9.39, 1.68)	(-18.08, -1.19)	(-4.32, 15.88)	
Week 8	LS Mean (SEM)	-8.09 (3.104)	-3.03 (4.829)	-5.06 (5.738)	0.3827
	95% CI for LS MEAN	(-14.35, -1.83)	(-12.76, 6.69)	(-16.62, 6.50)	
Week 12	LS Mean (SEM)	-3.27 (3.299)	-0.75 (4.992)	-2.52 (5.987)	0.6753
	95% CI for LS MEAN	(-9.90, 3.36)	(-10.79, 9.30)	(-14.56, 9.52)	

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties
Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.25 (5.393)	10.86 (8.737)	-13.11 (9.848)	0.1935
	95% CI for LS MEAN	(-13.29, 8.78)	(-6.95, 28.67)	(-33.26, 7.04)	
Week 4	LS Mean (SEM)	-2.25 (5.780)	5.34 (9.092)	-7.59 (10.405)	0.4719
	95% CI for LS MEAN	(-14.06, 9.56)	(-13.24, 23.92)	(-28.90, 13.73)	
Week 8	LS Mean (SEM)	-8.70 (4.260)	-1.32 (6.955)	-7.38 (7.500)	0.3400
	95% CI for LS MEAN	(-17.71, 0.32)	(-15.99, 13.35)	(-23.29, 8.53)	
Week 12	LS Mean (SEM)	-1.82 (5.260)	1.63 (8.204)	-3.45 (9.359)	0.7147
	95% CI for LS MEAN	(-12.57, 8.92)	(-15.14, 18.40)	(-22.61, 15.70)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.2.6
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties

Subgroup by treatment interaction p-value	0.1108
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:48:13

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue
Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-4.76 (4.344)	6.45 (6.509)	-11.21 (7.546)	0.1485
	95% CI for LS MEAN	(-13.66, 4.14)	(-6.84, 19.74)	(-26.66, 4.24)	
Week 4	LS Mean (SEM)	-10.05 (4.697)	11.64 (6.855)	-21.69 (8.066)	0.0121
	95% CI for LS MEAN	(-19.67, -0.43)	(-2.39, 25.68)	(-38.24, -5.15)	
Week 8	LS Mean (SEM)	-11.09 (4.376)	12.71 (7.213)	-23.80 (8.114)	0.0075
	95% CI for LS MEAN	(-20.17, -2.02)	(-2.13, 27.55)	(-40.58, -7.01)	
Week 12	LS Mean (SEM)	-9.46 (5.241)	14.98 (7.236)	-24.44 (8.697)	0.0092
	95% CI for LS MEAN	(-20.18, 1.26)	(0.13, 29.83)	(-42.29, -6.58)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue
Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-16.06 (3.336)	-3.72 (5.195)	-12.34 (6.118)	0.0506
	95% CI for LS MEAN	(-22.80, -9.32)	(-14.22, 6.78)	(-24.71, 0.03)	
Week 4	LS Mean (SEM)	-20.00 (3.068)	-5.31 (4.858)	-14.69 (5.679)	0.0128
	95% CI for LS MEAN	(-26.17, -13.84)	(-15.08, 4.46)	(-26.12, -3.27)	
Week 8	LS Mean (SEM)	-17.56 (3.523)	-11.87 (5.408)	-5.69 (6.375)	0.3775
	95% CI for LS MEAN	(-24.67, -10.45)	(-22.81, -0.92)	(-18.60, 7.22)	
Week 12	LS Mean (SEM)	-19.71 (2.944)	-11.66 (4.624)	-8.06 (5.411)	0.1437
	95% CI for LS MEAN	(-25.64, -13.79)	(-20.98, -2.34)	(-18.96, 2.85)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue

Subgroup by treatment interaction p-value	0.2062
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting
Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	2.39 (1.243)	1.70 (1.986)	0.70 (2.279)	0.7629
	95% CI for LS MEAN	(-0.18, 4.96)	(-2.40, 5.79)	(-4.02, 5.41)	
Week 4	LS Mean (SEM)	3.19 (1.567)	-0.16 (2.283)	3.34 (2.738)	0.2320
	95% CI for LS MEAN	(-0.02, 6.39)	(-4.82, 4.51)	(-2.26, 8.94)	
Week 8	LS Mean (SEM)	3.96 (5.538)	13.25 (9.649)	-9.28 (11.097)	0.4130
	95% CI for LS MEAN	(-7.63, 15.56)	(-6.91, 33.41)	(-32.49, 13.92)	
Week 12	LS Mean (SEM)	0.69 (1.739)	1.51 (2.214)	-0.82 (2.801)	0.7740
	95% CI for LS MEAN	(-2.95, 4.33)	(-3.14, 6.16)	(-6.70, 5.07)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting
Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	0.14 (2.098)	1.68 (3.297)	-1.54 (3.893)	0.6945
	95% CI for LS MEAN	(-4.07, 4.36)	(-4.95, 8.32)	(-9.37, 6.29)	
Week 4	LS Mean (SEM)	-0.36 (1.798)	-1.89 (2.849)	1.53 (3.348)	0.6494
	95% CI for LS MEAN	(-3.98, 3.26)	(-7.63, 3.85)	(-5.21, 8.28)	
Week 8	LS Mean (SEM)	-1.23 (2.017)	-3.55 (3.116)	2.32 (3.674)	0.5326
	95% CI for LS MEAN	(-5.36, 2.91)	(-9.97, 2.86)	(-5.24, 9.89)	
Week 12	LS Mean (SEM)	0.42 (2.384)	1.68 (3.753)	-1.26 (4.429)	0.7778
	95% CI for LS MEAN	(-4.39, 5.23)	(-5.90, 9.26)	(-10.20, 7.68)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting

Subgroup by treatment interaction p-value	0.6137
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain
Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-3.48 (4.487)	1.95 (7.426)	-5.44 (8.566)	0.5278
	95% CI for LS MEAN	(-12.45, 5.48)	(-12.86, 16.77)	(-22.53, 11.66)	
Week 4	LS Mean (SEM)	1.28 (4.487)	-1.00 (6.644)	2.28 (7.902)	0.7742
	95% CI for LS MEAN	(-7.69, 10.25)	(-14.28, 12.29)	(-13.52, 18.07)	
Week 8	LS Mean (SEM)	-0.82 (4.616)	3.33 (7.083)	-4.15 (8.364)	0.6210
	95% CI for LS MEAN	(-10.04, 8.39)	(-10.81, 17.46)	(-20.84, 12.53)	
Week 12	LS Mean (SEM)	-1.22 (4.594)	-0.49 (6.903)	-0.73 (8.200)	0.9294
	95% CI for LS MEAN	(-10.40, 7.95)	(-14.28, 13.30)	(-17.10, 15.65)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain
Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.54 (2.931)	5.80 (4.548)	-7.34 (5.347)	0.1765
	95% CI for LS MEAN	(-7.44, 4.36)	(-3.36, 14.96)	(-18.11, 3.43)	
Week 4	LS Mean (SEM)	-3.23 (2.954)	5.80 (4.665)	-9.03 (5.445)	0.1043
	95% CI for LS MEAN	(-9.17, 2.72)	(-3.59, 15.20)	(-20.00, 1.94)	
Week 8	LS Mean (SEM)	1.46 (4.084)	-1.16 (6.144)	2.62 (7.303)	0.7220
	95% CI for LS MEAN	(-6.83, 9.76)	(-13.69, 11.37)	(-12.27, 17.51)	
Week 12	LS Mean (SEM)	-1.91 (3.591)	9.37 (5.671)	-11.28 (6.650)	0.0969
	95% CI for LS MEAN	(-9.13, 5.32)	(-2.05, 20.80)	(-24.68, 2.12)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain

Subgroup by treatment interaction p-value	0.3702
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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:48:15

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea
Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-6.56 (5.205)	-3.92 (8.136)	-2.64 (9.347)	0.7800
	95% CI for LS MEAN	(-17.27, 4.16)	(-20.55, 12.72)	(-21.85, 16.57)	
Week 4	LS Mean (SEM)	-16.08 (5.232)	-7.42 (7.824)	-8.66 (9.133)	0.3514
	95% CI for LS MEAN	(-26.79, -5.37)	(-23.42, 8.57)	(-27.36, 10.05)	
Week 8	LS Mean (SEM)	-11.82 (4.713)	11.90 (7.636)	-23.72 (8.607)	0.0106
	95% CI for LS MEAN	(-21.55, -2.10)	(-3.72, 27.52)	(-41.42, -6.02)	
Week 12	LS Mean (SEM)	-14.98 (5.722)	-0.76 (8.051)	-14.23 (9.619)	0.1519
	95% CI for LS MEAN	(-26.75, -3.22)	(-17.34, 15.83)	(-34.06, 5.60)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:48:15

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea
Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-18.94 (3.533)	3.21 (5.545)	-22.15 (6.494)	0.0014
	95% CI for LS MEAN	(-26.06, -11.82)	(-7.97, 14.40)	(-35.25, -9.05)	
Week 4	LS Mean (SEM)	-25.56 (3.833)	5.59 (6.068)	-31.16 (7.097)	<0.0001
	95% CI for LS MEAN	(-33.27, -17.86)	(-6.61, 17.80)	(-45.43, -16.88)	
Week 8	LS Mean (SEM)	-22.22 (3.794)	-11.43 (5.861)	-10.79 (6.889)	0.1256
	95% CI for LS MEAN	(-29.87, -14.56)	(-23.29, 0.43)	(-24.73, 3.16)	
Week 12	LS Mean (SEM)	-21.54 (3.631)	-8.69 (5.689)	-12.85 (6.660)	0.0604
	95% CI for LS MEAN	(-28.86, -14.22)	(-20.16, 2.78)	(-26.28, 0.59)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea

Subgroup by treatment interaction p-value	0.4047
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia
Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-7.40 (5.685)	0.99 (8.719)	-8.39 (10.053)	0.4111
	95% CI for LS MEAN	(-19.06, 4.26)	(-16.80, 18.79)	(-28.99, 12.21)	
Week 4	LS Mean (SEM)	-1.05 (6.718)	-3.84 (9.772)	2.80 (11.607)	0.8114
	95% CI for LS MEAN	(-14.80, 12.71)	(-23.85, 16.16)	(-21.00, 26.60)	
Week 8	LS Mean (SEM)	-11.94 (5.327)	-15.29 (9.194)	3.35 (10.221)	0.7464
	95% CI for LS MEAN	(-23.14, -0.74)	(-34.34, 3.76)	(-17.97, 24.67)	
Week 12	LS Mean (SEM)	-8.89 (7.140)	2.82 (9.532)	-11.72 (11.651)	0.3238
	95% CI for LS MEAN	(-23.52, 5.73)	(-16.75, 22.40)	(-35.66, 12.23)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia
Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.06 (3.806)	0.86 (5.916)	-2.92 (6.961)	0.6770
	95% CI for LS MEAN	(-9.75, 5.63)	(-11.11, 12.84)	(-17.01, 11.17)	
Week 4	LS Mean (SEM)	-7.34 (3.894)	-1.52 (6.163)	-5.83 (7.215)	0.4234
	95% CI for LS MEAN	(-15.17, 0.48)	(-13.91, 10.88)	(-20.34, 8.69)	
Week 8	LS Mean (SEM)	-11.60 (3.980)	0.19 (6.100)	-11.79 (7.198)	0.1092
	95% CI for LS MEAN	(-19.62, -3.58)	(-12.13, 12.51)	(-26.32, 2.75)	
Week 12	LS Mean (SEM)	-8.77 (3.244)	3.24 (5.061)	-12.01 (5.918)	0.0487
	95% CI for LS MEAN	(-15.31, -2.23)	(-6.97, 13.45)	(-23.95, -0.07)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:48:15

Data cutoff date of 31Mar2023
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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia

Subgroup by treatment interaction p-value	0.5539
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss
Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	3.79 (4.188)	5.67 (6.338)	-1.88 (7.290)	0.7983
	95% CI for LS MEAN	(-4.83, 12.40)	(-7.30, 18.64)	(-16.86, 13.09)	
Week 4	LS Mean (SEM)	-2.56 (3.991)	2.03 (5.814)	-4.59 (6.771)	0.5036
	95% CI for LS MEAN	(-10.75, 5.63)	(-9.90, 13.96)	(-18.50, 9.31)	
Week 8	LS Mean (SEM)	4.71 (6.458)	7.69 (9.836)	-2.97 (11.589)	0.7999
	95% CI for LS MEAN	(-8.69, 18.12)	(-12.56, 27.93)	(-26.97, 21.02)	
Week 12	LS Mean (SEM)	5.83 (6.751)	2.03 (9.428)	3.80 (11.430)	0.7420
	95% CI for LS MEAN	(-8.00, 19.67)	(-17.37, 21.43)	(-19.72, 27.33)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss
Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-3.17 (2.721)	3.13 (4.319)	-6.30 (5.107)	0.2234
	95% CI for LS MEAN	(-8.64, 2.30)	(-5.56, 11.82)	(-16.57, 3.97)	
Week 4	LS Mean (SEM)	-6.12 (2.613)	-4.01 (4.177)	-2.10 (4.928)	0.6711
	95% CI for LS MEAN	(-11.37, -0.87)	(-12.41, 4.38)	(-12.01, 7.80)	
Week 8	LS Mean (SEM)	0.36 (3.203)	-0.27 (4.979)	0.63 (5.912)	0.9154
	95% CI for LS MEAN	(-6.08, 6.80)	(-10.30, 9.76)	(-11.28, 12.54)	
Week 12	LS Mean (SEM)	-6.79 (3.017)	3.13 (4.787)	-9.92 (5.658)	0.0871
	95% CI for LS MEAN	(-12.89, -0.70)	(-6.54, 12.80)	(-21.36, 1.51)	

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Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss

Subgroup by treatment interaction p-value	0.8056
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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation
Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-4.12 (4.488)	6.06 (6.802)	-10.18 (7.845)	0.2056
	95% CI for LS MEAN	(-13.34, 5.11)	(-7.84, 19.97)	(-26.28, 5.93)	
Week 4	LS Mean (SEM)	-5.70 (4.301)	16.50 (6.295)	-22.21 (7.333)	0.0053
	95% CI for LS MEAN	(-14.52, 3.11)	(3.60, 29.40)	(-37.25, -7.16)	
Week 8	LS Mean (SEM)	-10.67 (5.958)	29.84 (8.811)	-40.50 (10.457)	0.0014
	95% CI for LS MEAN	(-23.35, 2.02)	(11.30, 48.38)	(-62.72, -18.28)	
Week 12	LS Mean (SEM)	-9.75 (4.215)	13.17 (6.023)	-22.91 (7.052)	0.0032
	95% CI for LS MEAN	(-18.39, -1.10)	(0.79, 25.55)	(-37.41, -8.42)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:48:15

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation
Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.35 (3.429)	8.01 (5.363)	-9.37 (6.285)	0.1431
	95% CI for LS MEAN	(-8.25, 5.54)	(-2.79, 18.81)	(-22.02, 3.29)	
Week 4	LS Mean (SEM)	-0.04 (3.696)	5.63 (5.851)	-5.67 (6.846)	0.4116
	95% CI for LS MEAN	(-7.47, 7.39)	(-6.14, 17.40)	(-19.45, 8.10)	
Week 8	LS Mean (SEM)	-2.62 (3.224)	3.81 (4.944)	-6.43 (5.792)	0.2730
	95% CI for LS MEAN	(-9.12, 3.88)	(-6.17, 13.80)	(-18.13, 5.26)	
Week 12	LS Mean (SEM)	1.20 (3.464)	5.63 (5.430)	-4.43 (6.359)	0.4900
	95% CI for LS MEAN	(-5.77, 8.18)	(-5.31, 16.57)	(-17.24, 8.39)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation

Subgroup by treatment interaction p-value	0.0633
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:48:15

Data cutoff date of 31Mar2023
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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea
Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-5.30 (1.929)	-10.01 (3.114)	4.71 (3.630)	0.2050
	95% CI for LS MEAN	(-9.26, -1.34)	(-16.36, -3.65)	(-2.72, 12.13)	
Week 4	LS Mean (SEM)	-3.71 (2.532)	-9.75 (3.703)	6.04 (4.481)	0.1883
	95% CI for LS MEAN	(-8.89, 1.47)	(-17.32, -2.18)	(-3.13, 15.20)	
Week 8	LS Mean (SEM)	5.31 (5.714)	4.66 (9.527)	0.65 (11.076)	0.9541
	95% CI for LS MEAN	(-6.67, 17.29)	(-15.24, 24.56)	(-22.50, 23.79)	
Week 12	LS Mean (SEM)	-3.99 (4.307)	-6.42 (5.651)	2.43 (7.102)	0.7355
	95% CI for LS MEAN	(-12.85, 4.87)	(-18.07, 5.23)	(-12.20, 17.06)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:48:15

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea
Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	2.13 (3.364)	1.52 (5.320)	0.61 (6.270)	0.9223
	95% CI for LS MEAN	(-4.64, 8.90)	(-9.20, 12.23)	(-12.01, 13.24)	
Week 4	LS Mean (SEM)	0.22 (2.409)	-0.86 (3.827)	1.09 (4.487)	0.8095
	95% CI for LS MEAN	(-4.62, 5.07)	(-8.56, 6.83)	(-7.94, 10.12)	
Week 8	LS Mean (SEM)	-0.23 (1.862)	-3.24 (2.887)	3.00 (3.384)	0.3800
	95% CI for LS MEAN	(-3.98, 3.52)	(-9.06, 2.59)	(-3.83, 9.84)	
Week 12	LS Mean (SEM)	0.27 (1.868)	-3.25 (2.932)	3.51 (3.429)	0.3109
	95% CI for LS MEAN	(-3.49, 4.03)	(-9.15, 2.66)	(-3.39, 10.42)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:48:15

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea

Subgroup by treatment interaction p-value	0.7014
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:48:15

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties
Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.44 (5.711)	3.28 (8.524)	-4.73 (9.892)	0.6357
	95% CI for LS MEAN	(-13.06, 10.18)	(-14.02, 20.59)	(-24.81, 15.36)	
Week 4	LS Mean (SEM)	0.14 (5.711)	-4.35 (8.357)	4.49 (9.769)	0.6485
	95% CI for LS MEAN	(-11.48, 11.76)	(-21.36, 12.66)	(-15.37, 24.36)	
Week 8	LS Mean (SEM)	-10.45 (6.057)	-1.74 (9.462)	-8.71 (10.896)	0.4281
	95% CI for LS MEAN	(-22.69, 1.80)	(-20.76, 17.29)	(-30.63, 13.21)	
Week 12	LS Mean (SEM)	-1.65 (5.880)	-1.02 (8.357)	-0.64 (9.869)	0.9488
	95% CI for LS MEAN	(-13.58, 10.27)	(-18.02, 15.99)	(-20.68, 19.41)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:48:15

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties
Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.85 (2.685)	3.66 (4.244)	-5.51 (4.956)	0.2725
	95% CI for LS MEAN	(-7.25, 3.55)	(-4.89, 12.20)	(-15.49, 4.48)	
Week 4	LS Mean (SEM)	-3.70 (3.006)	-1.11 (4.766)	-2.60 (5.577)	0.6437
	95% CI for LS MEAN	(-9.75, 2.35)	(-10.70, 8.49)	(-13.83, 8.63)	
Week 8	LS Mean (SEM)	-3.85 (3.462)	3.61 (5.369)	-7.46 (6.334)	0.2452
	95% CI for LS MEAN	(-10.81, 3.11)	(-7.21, 14.43)	(-20.22, 5.30)	
Week 12	LS Mean (SEM)	-2.24 (3.164)	3.66 (4.985)	-5.90 (5.849)	0.3184
	95% CI for LS MEAN	(-8.60, 4.12)	(-6.37, 13.69)	(-17.67, 5.87)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:15

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Table GERMANY.2.5.2.7
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties

Subgroup by treatment interaction p-value	0.9024
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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Data cutoff date of 31Mar2023
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Table GERMANY.2.5.2.8
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue
Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-23.31 (13.966)	4.43 (9.534)	-27.74 (19.873)	0.1951
	95% CI for LS MEAN	(-55.00, 8.39)	(-17.29, 26.15)	(-72.46, 16.98)	
Week 4	LS Mean (SEM)	-26.08 (14.854)	7.21 (11.919)	-33.29 (21.718)	0.1513
	95% CI for LS MEAN	(-58.68, 6.51)	(-19.42, 33.83)	(-80.63, 14.05)	
Week 8	LS Mean (SEM)	-21.92 (14.055)	-1.13 (9.791)	-20.79 (20.060)	0.3246
	95% CI for LS MEAN	(-53.61, 9.78)	(-23.01, 20.76)	(-65.56, 23.98)	
Week 12	LS Mean (SEM)	-27.47 (14.385)	4.43 (10.707)	-31.90 (20.750)	0.1528
	95% CI for LS MEAN	(-59.49, 4.54)	(-19.53, 28.39)	(-77.65, 13.84)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:48:18

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Table GERMANY.2.5.2.8
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue
Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-13.16 (2.998)	0.64 (4.680)	-13.80 (5.475)	0.0143
	95% CI for LS MEAN	(-19.15, -7.17)	(-8.71, 9.99)	(-24.75, -2.86)	
Week 4	LS Mean (SEM)	-17.92 (2.897)	1.34 (4.488)	-19.26 (5.258)	0.0005
	95% CI for LS MEAN	(-23.70, -12.14)	(-7.62, 10.30)	(-29.76, -8.76)	
Week 8	LS Mean (SEM)	-18.18 (3.274)	-0.90 (5.187)	-17.28 (6.034)	0.0061
	95% CI for LS MEAN	(-24.75, -11.61)	(-11.30, 9.50)	(-29.39, -5.16)	
Week 12	LS Mean (SEM)	-16.83 (3.146)	-0.88 (4.768)	-15.95 (5.632)	0.0062
	95% CI for LS MEAN	(-23.11, -10.55)	(-10.41, 8.65)	(-27.20, -4.69)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:18

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Table GERMANY.2.5.2.8

Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue

Subgroup by treatment interaction p-value	0.2011
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Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.8
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting
Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.25 (1.433)	0.10 (1.322)	-0.36 (2.080)	0.8663
	95% CI for LS MEAN	(-3.47, 2.96)	(-2.61, 2.81)	(-4.84, 4.13)	
Week 4	LS Mean (SEM)	-0.25 (1.433)	0.10 (1.322)	-0.36 (2.080)	0.8663
	95% CI for LS MEAN	(-3.47, 2.96)	(-2.61, 2.81)	(-4.84, 4.13)	
Week 8	LS Mean (SEM)	1.83 (1.433)	0.10 (1.322)	1.73 (2.080)	0.4213
	95% CI for LS MEAN	(-1.39, 5.05)	(-2.61, 2.81)	(-2.76, 6.21)	
Week 12	LS Mean (SEM)	-0.25 (1.433)	0.10 (1.322)	-0.36 (2.080)	0.8663
	95% CI for LS MEAN	(-3.47, 2.96)	(-2.61, 2.81)	(-4.84, 4.13)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.8
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting
Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	0.93 (1.687)	2.67 (2.632)	-1.73 (3.093)	0.5774
	95% CI for LS MEAN	(-2.43, 4.30)	(-2.59, 7.92)	(-7.91, 4.45)	
Week 4	LS Mean (SEM)	1.12 (1.484)	-0.89 (2.278)	2.00 (2.682)	0.4583
	95% CI for LS MEAN	(-1.85, 4.08)	(-5.44, 3.67)	(-3.36, 7.36)	
Week 8	LS Mean (SEM)	1.51 (2.536)	5.06 (4.023)	-3.55 (4.726)	0.4566
	95% CI for LS MEAN	(-3.59, 6.61)	(-3.02, 13.14)	(-13.05, 5.96)	
Week 12	LS Mean (SEM)	1.73 (2.114)	2.45 (3.177)	-0.72 (3.790)	0.8499
	95% CI for LS MEAN	(-2.51, 5.97)	(-3.94, 8.83)	(-8.34, 6.89)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.8

Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting

Subgroup by treatment interaction p-value	0.9271
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain
Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM) 95% CI for LS MEAN				
Week 4	LS Mean (SEM) 95% CI for LS MEAN				
Week 8	LS Mean (SEM) 95% CI for LS MEAN				
Week 12	LS Mean (SEM) 95% CI for LS MEAN				

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain
Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.66 (2.500)	3.55 (3.986)	-6.21 (4.628)	0.1840
	95% CI for LS MEAN	(-7.65, 2.33)	(-4.40, 11.51)	(-15.46, 3.03)	
Week 4	LS Mean (SEM)	-2.63 (2.509)	1.96 (3.872)	-4.58 (4.531)	0.3156
	95% CI for LS MEAN	(-7.64, 2.39)	(-5.78, 9.69)	(-13.64, 4.47)	
Week 8	LS Mean (SEM)	-1.21 (4.152)	-3.84 (6.860)	2.63 (7.964)	0.7433
	95% CI for LS MEAN	(-9.59, 7.16)	(-17.67, 9.99)	(-13.44, 18.69)	
Week 12	LS Mean (SEM)	-3.26 (3.261)	3.69 (5.013)	-6.95 (5.916)	0.2444
	95% CI for LS MEAN	(-9.77, 3.24)	(-6.33, 13.70)	(-18.77, 4.87)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.8

Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain

Subgroup by treatment interaction p-value	0.8608
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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea
Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-12.50 (16.278)	3.33 (12.439)	-15.83 (23.079)	0.5052
	95% CI for LS MEAN	(-48.20, 23.20)	(-24.29, 30.95)	(-65.91, 34.25)	
Week 4	LS Mean (SEM)	-20.83 (17.029)	3.33 (14.308)	-24.17 (24.650)	0.3444
	95% CI for LS MEAN	(-57.70, 16.04)	(-28.58, 35.24)	(-77.29, 28.96)	
Week 8	LS Mean (SEM)	-16.67 (16.501)	-5.00 (13.012)	-11.67 (23.548)	0.6287
	95% CI for LS MEAN	(-52.69, 19.36)	(-33.97, 23.97)	(-62.64, 39.31)	
Week 12	LS Mean (SEM)	-16.67 (15.140)	-5.00 (9.121)	-11.67 (20.624)	0.5856
	95% CI for LS MEAN	(-51.36, 18.02)	(-25.78, 15.78)	(-58.43, 35.10)	

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Table GERMANY.2.5.2.8
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea
Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-14.64 (3.195)	0.57 (5.019)	-15.21 (5.834)	0.0115
	95% CI for LS MEAN	(-21.03, -8.25)	(-9.47, 10.60)	(-26.88, -3.54)	
Week 4	LS Mean (SEM)	-22.27 (3.343)	0.15 (5.179)	-22.43 (6.059)	0.0004
	95% CI for LS MEAN	(-28.94, -15.60)	(-10.18, 10.49)	(-34.52, -10.33)	
Week 8	LS Mean (SEM)	-18.89 (3.542)	-5.58 (5.623)	-13.32 (6.533)	0.0465
	95% CI for LS MEAN	(-25.99, -11.80)	(-16.84, 5.69)	(-26.42, -0.22)	
Week 12	LS Mean (SEM)	-19.45 (3.538)	-4.85 (5.273)	-14.61 (6.248)	0.0229
	95% CI for LS MEAN	(-26.53, -12.38)	(-15.40, 5.71)	(-27.12, -2.10)	

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea

Subgroup by treatment interaction p-value	0.7837
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:48:18

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Table GERMANY.2.5.2.8
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia
Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-23.16 (10.505)	11.87 (11.580)	-35.03 (16.603)	0.0562
	95% CI for LS MEAN	(-45.77, -0.54)	(-14.17, 37.91)	(-71.15, 1.09)	
Week 4	LS Mean (SEM)	-23.16 (9.392)	3.54 (9.475)	-26.70 (14.464)	0.0870
	95% CI for LS MEAN	(-43.28, -3.03)	(-17.51, 24.59)	(-57.83, 4.44)	
Week 8	LS Mean (SEM)	-27.32 (8.363)	11.87 (7.296)	-39.20 (12.425)	0.0077
	95% CI for LS MEAN	(-45.50, -9.15)	(-4.25, 27.99)	(-66.07, -12.32)	
Week 12	LS Mean (SEM)	-27.32 (8.363)	11.87 (7.296)	-39.20 (12.425)	0.0077
	95% CI for LS MEAN	(-45.50, -9.15)	(-4.25, 27.99)	(-66.07, -12.32)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:48:18

Data cutoff date of 31Mar2023
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Table GERMANY.2.5.2.8
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia
Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-4.85 (3.606)	-1.44 (5.617)	-3.41 (6.570)	0.6056
	95% CI for LS MEAN	(-12.06, 2.36)	(-12.67, 9.78)	(-16.55, 9.73)	
Week 4	LS Mean (SEM)	-5.84 (3.959)	-3.66 (6.133)	-2.18 (7.209)	0.7633
	95% CI for LS MEAN	(-13.74, 2.06)	(-15.90, 8.58)	(-16.57, 12.21)	
Week 8	LS Mean (SEM)	-12.88 (3.677)	-8.95 (5.842)	-3.93 (6.773)	0.5650
	95% CI for LS MEAN	(-20.27, -5.49)	(-20.69, 2.79)	(-17.55, 9.70)	
Week 12	LS Mean (SEM)	-9.52 (3.589)	1.34 (5.354)	-10.86 (6.341)	0.0918
	95% CI for LS MEAN	(-16.68, -2.36)	(-9.36, 12.04)	(-23.53, 1.81)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.8

Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia

Subgroup by treatment interaction p-value 0.4848

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.8
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss
Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	2.49 (11.782)	2.86 (6.906)	-0.37 (15.438)	0.9820
	95% CI for LS MEAN	(-26.66, 31.64)	(-29.71, 35.43)	(-43.27, 42.53)	
Week 4	LS Mean (SEM)	2.49 (13.096)	-5.47 (10.632)	7.96 (18.340)	0.6800
	95% CI for LS MEAN	(-28.18, 33.16)	(-35.01, 24.07)	(-37.40, 53.33)	
Week 8	LS Mean (SEM)	10.82 (12.216)	2.86 (8.279)	7.96 (16.419)	0.6387
	95% CI for LS MEAN	(-16.16, 37.80)	(-17.09, 22.81)	(-28.86, 44.79)	
Week 12	LS Mean (SEM)	-1.68 (14.579)	11.19 (13.970)	-12.87 (21.436)	0.5703
	95% CI for LS MEAN	(-35.95, 32.59)	(-25.26, 47.65)	(-65.36, 39.62)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:48:18

Data cutoff date of 31Mar2023
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Table GERMANY.2.5.2.8
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss
Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.71 (2.566)	3.50 (4.018)	-4.21 (4.732)	0.3771
	95% CI for LS MEAN	(-5.83, 4.41)	(-4.52, 11.51)	(-13.65, 5.23)	
Week 4	LS Mean (SEM)	-5.58 (2.459)	-1.75 (3.791)	-3.82 (4.487)	0.3971
	95% CI for LS MEAN	(-10.48, -0.67)	(-9.32, 5.82)	(-12.78, 5.13)	
Week 8	LS Mean (SEM)	0.90 (3.399)	1.82 (5.381)	-0.92 (6.338)	0.8850
	95% CI for LS MEAN	(-5.93, 7.72)	(-8.98, 12.62)	(-13.66, 11.81)	
Week 12	LS Mean (SEM)	-1.40 (3.412)	-0.09 (5.186)	-1.31 (6.184)	0.8324
	95% CI for LS MEAN	(-8.21, 5.41)	(-10.45, 10.28)	(-13.67, 11.05)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:48:18

Data cutoff date of 31Mar2023
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Table GERMANY.2.5.2.8

Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss

Subgroup by treatment interaction p-value	0.9358
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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.8
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation
Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-8.28 (11.649)	-3.01 (8.870)	-5.27 (16.016)	0.7498
	95% CI for LS MEAN	(-35.03, 18.48)	(-22.26, 16.25)	(-41.50, 30.96)	
Week 4	LS Mean (SEM)	0.06 (11.649)	5.32 (8.870)	-5.27 (16.016)	0.7498
	95% CI for LS MEAN	(-26.70, 26.82)	(-13.93, 24.58)	(-41.50, 30.96)	
Week 8	LS Mean (SEM)	-12.44 (11.649)	-3.01 (8.870)	-9.43 (16.016)	0.5703
	95% CI for LS MEAN	(-39.20, 14.32)	(-22.26, 16.25)	(-45.66, 26.79)	
Week 12	LS Mean (SEM)	-4.11 (11.649)	-3.01 (8.870)	-1.10 (16.016)	0.9467
	95% CI for LS MEAN	(-30.87, 22.65)	(-22.26, 16.25)	(-37.33, 35.13)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:48:18

Data cutoff date of 31Mar2023
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Table GERMANY.2.5.2.8
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation
Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.41 (3.095)	9.13 (4.801)	-10.54 (5.610)	0.0648
	95% CI for LS MEAN	(-7.59, 4.77)	(-0.45, 18.71)	(-21.75, 0.67)	
Week 4	LS Mean (SEM)	-2.42 (3.108)	10.66 (4.797)	-13.09 (5.616)	0.0229
	95% CI for LS MEAN	(-8.63, 3.78)	(1.08, 20.24)	(-24.31, -1.87)	
Week 8	LS Mean (SEM)	-4.46 (3.029)	11.71 (4.819)	-16.17 (5.557)	0.0054
	95% CI for LS MEAN	(-10.54, 1.62)	(2.04, 21.37)	(-27.33, -5.01)	
Week 12	LS Mean (SEM)	-1.66 (3.090)	10.66 (4.629)	-12.32 (5.462)	0.0276
	95% CI for LS MEAN	(-7.83, 4.51)	(1.41, 19.92)	(-23.24, -1.41)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation

Subgroup by treatment interaction p-value	0.3765
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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea
Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.44 (8.859)	9.09 (6.814)	-2.65 (12.226)	0.8322
	95% CI for LS MEAN	(-13.35, 26.23)	(-5.32, 23.50)	(-29.53, 24.23)	
Week 4	LS Mean (SEM)	14.77 (8.859)	9.09 (6.814)	5.68 (12.226)	0.6511
	95% CI for LS MEAN	(-5.02, 34.56)	(-5.32, 23.50)	(-21.20, 32.56)	
Week 8	LS Mean (SEM)	10.61 (8.859)	9.09 (6.814)	1.52 (12.226)	0.9036
	95% CI for LS MEAN	(-9.18, 30.40)	(-5.32, 23.50)	(-25.36, 28.39)	
Week 12	LS Mean (SEM)	10.61 (8.859)	9.09 (6.814)	1.52 (12.226)	0.9036
	95% CI for LS MEAN	(-9.18, 30.40)	(-5.32, 23.50)	(-25.36, 28.39)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:48:18

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.8
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea
Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.27 (2.512)	-5.40 (3.964)	4.14 (4.673)	0.3792
	95% CI for LS MEAN	(-6.28, 3.75)	(-13.32, 2.51)	(-5.19, 13.47)	
Week 4	LS Mean (SEM)	-3.39 (1.860)	-7.17 (2.879)	3.77 (3.402)	0.2712
	95% CI for LS MEAN	(-7.10, 0.32)	(-12.91, -1.42)	(-3.02, 10.57)	
Week 8	LS Mean (SEM)	0.49 (2.566)	-4.38 (4.158)	4.87 (4.861)	0.3206
	95% CI for LS MEAN	(-4.66, 5.64)	(-12.72, 3.95)	(-4.88, 14.63)	
Week 12	LS Mean (SEM)	-2.07 (2.089)	-7.17 (3.072)	5.10 (3.689)	0.1722
	95% CI for LS MEAN	(-6.25, 2.11)	(-13.31, -1.02)	(-2.28, 12.48)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:18

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.8

Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea

Subgroup by treatment interaction p-value	0.1502
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.8
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties
Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-12.64 (8.790)	-9.94 (7.246)	-2.70 (12.471)	0.8324
	95% CI for LS MEAN	(-32.24, 6.95)	(-24.99, 5.10)	(-29.85, 24.46)	
Week 4	LS Mean (SEM)	-12.64 (8.790)	-9.94 (7.246)	-2.70 (12.471)	0.8324
	95% CI for LS MEAN	(-32.24, 6.95)	(-24.99, 5.10)	(-29.85, 24.46)	
Week 8	LS Mean (SEM)	-25.14 (8.790)	-9.94 (7.246)	-15.20 (12.471)	0.2463
	95% CI for LS MEAN	(-44.74, -5.55)	(-24.99, 5.10)	(-42.35, 11.96)	
Week 12	LS Mean (SEM)	-16.81 (8.790)	-9.94 (7.246)	-6.86 (12.471)	0.5921
	95% CI for LS MEAN	(-36.40, 2.79)	(-24.99, 5.10)	(-34.02, 20.29)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:18

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.8
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties
Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.64 (2.942)	5.44 (4.628)	-7.08 (5.394)	0.1941
	95% CI for LS MEAN	(-7.51, 4.24)	(-3.80, 14.68)	(-17.85, 3.70)	
Week 4	LS Mean (SEM)	-2.32 (3.171)	-1.80 (4.929)	-0.51 (5.783)	0.9294
	95% CI for LS MEAN	(-8.65, 4.01)	(-11.64, 8.03)	(-12.06, 11.03)	
Week 8	LS Mean (SEM)	-4.30 (2.626)	2.26 (4.185)	-6.56 (4.805)	0.1793
	95% CI for LS MEAN	(-9.59, 0.99)	(-6.17, 10.69)	(-16.26, 3.13)	
Week 12	LS Mean (SEM)	-1.36 (3.139)	3.20 (4.792)	-4.56 (5.647)	0.4222
	95% CI for LS MEAN	(-7.62, 4.89)	(-6.37, 12.76)	(-15.83, 6.71)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:18

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Table GERMANY.2.5.2.8

Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties

Subgroup by treatment interaction p-value	0.4770
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue
Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-10.47 (3.233)	-8.27 (4.534)	-2.20 (5.210)	0.6755
	95% CI for LS MEAN	(-17.07, -3.87)	(-17.53, 1.00)	(-12.87, 8.46)	
Week 4	LS Mean (SEM)	-11.99 (4.084)	-1.60 (5.853)	-10.38 (6.861)	0.1408
	95% CI for LS MEAN	(-20.30, -3.67)	(-13.54, 10.34)	(-24.41, 3.64)	
Week 8	LS Mean (SEM)	-10.63 (3.625)	-12.71 (4.962)	2.08 (5.846)	0.7248
	95% CI for LS MEAN	(-18.03, -3.23)	(-22.88, -2.54)	(-9.92, 14.08)	
Week 12	LS Mean (SEM)	-15.49 (3.869)	-9.38 (5.441)	-6.11 (6.376)	0.3472
	95% CI for LS MEAN	(-23.41, -7.57)	(-20.58, 1.82)	(-19.26, 7.04)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:21

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue
White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-13.30 (3.499)	1.63 (5.918)	-14.92 (6.861)	0.0367
	95% CI for LS MEAN	(-20.42, -6.18)	(-10.40, 13.65)	(-28.87, -0.98)	
Week 4	LS Mean (SEM)	-18.70 (3.311)	0.19 (5.537)	-18.89 (6.441)	0.0060
	95% CI for LS MEAN	(-25.44, -11.97)	(-11.07, 11.45)	(-32.00, -5.79)	
Week 8	LS Mean (SEM)	-15.40 (4.496)	4.17 (7.712)	-19.56 (8.918)	0.0364
	95% CI for LS MEAN	(-24.60, -6.19)	(-11.59, 19.93)	(-37.80, -1.33)	
Week 12	LS Mean (SEM)	-14.16 (3.428)	-6.47 (5.548)	-7.69 (6.510)	0.2478
	95% CI for LS MEAN	(-21.19, -7.14)	(-17.86, 4.92)	(-21.05, 5.67)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:21

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue
Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-14.55 (11.680)	19.27 (14.421)	-33.82 (17.614)	0.0671
	95% CI for LS MEAN	(-38.68, 9.58)	(-10.52, 49.07)	(-70.22, 2.58)	
Week 4	LS Mean (SEM)	-24.07 (11.678)	10.94 (14.421)	-35.01 (17.613)	0.0586
	95% CI for LS MEAN	(-48.20, 0.05)	(-18.86, 40.74)	(-71.41, 1.38)	
Week 8	LS Mean (SEM)	-26.90 (13.886)	59.53 (17.620)	-86.43 (17.701)	<0.0001
	95% CI for LS MEAN	(-55.56, 1.77)	(23.17, 95.89)	(-122.99, -49.87)	
Week 12	LS Mean (SEM)	-25.66 (11.672)	33.16 (14.435)	-58.82 (17.635)	0.0028
	95% CI for LS MEAN	(-49.77, -1.55)	(3.34, 62.99)	(-95.27, -22.38)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Fatigue

Subgroup by treatment interaction p-value	0.0025
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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting
Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.16 (0.780)	-2.19 (1.147)	1.03 (1.379)	0.4599
	95% CI for LS MEAN	(-2.75, 0.44)	(-4.54, 0.16)	(-1.79, 3.86)	
Week 4	LS Mean (SEM)	-1.16 (1.761)	-2.19 (2.609)	1.03 (3.144)	0.7447
	95% CI for LS MEAN	(-4.76, 2.44)	(-7.52, 3.14)	(-5.39, 7.46)	
Week 8	LS Mean (SEM)	-1.36 (2.536)	-0.52 (3.666)	-0.84 (4.454)	0.8530
	95% CI for LS MEAN	(-6.62, 3.90)	(-8.15, 7.10)	(-10.09, 8.42)	
Week 12	LS Mean (SEM)	1.43 (2.222)	-5.52 (3.262)	6.95 (3.943)	0.0907
	95% CI for LS MEAN	(-3.16, 6.01)	(-12.26, 1.21)	(-1.19, 15.09)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting
White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	2.41 (1.926)	1.38 (3.274)	1.03 (3.794)	0.7877
	95% CI for LS MEAN	(-1.50, 6.33)	(-5.27, 8.03)	(-6.68, 8.74)	
Week 4	LS Mean (SEM)	3.91 (1.846)	-0.41 (3.100)	4.31 (3.603)	0.2393
	95% CI for LS MEAN	(0.16, 7.66)	(-6.70, 5.89)	(-3.00, 11.63)	
Week 8	LS Mean (SEM)	2.05 (3.374)	0.89 (5.835)	1.16 (6.737)	0.8646
	95% CI for LS MEAN	(-4.87, 8.97)	(-11.09, 12.87)	(-12.66, 14.98)	
Week 12	LS Mean (SEM)	1.27 (1.493)	1.26 (2.402)	0.01 (2.823)	0.9978
	95% CI for LS MEAN	(-1.91, 4.45)	(-3.88, 6.41)	(-6.03, 6.05)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting
Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	0.88 (10.341)	9.85 (12.244)	-8.96 (14.938)	0.5809
	95% CI for LS MEAN	(-26.99, 28.76)	(-23.94, 43.63)	(-50.47, 32.55)	
Week 4	LS Mean (SEM)	-3.88 (7.746)	-2.65 (8.231)	-1.22 (9.697)	0.9077
	95% CI for LS MEAN	(-27.03, 19.27)	(-28.70, 23.39)	(-32.52, 30.07)	
Week 8	LS Mean (SEM)	-0.01 (11.557)	-9.62 (34.342)	9.61 (35.769)	0.8330
	95% CI for LS MEAN	(-93.33, 93.30)	(-197.90, 178.66)	(-444.88, 464.09)	
Week 12	LS Mean (SEM)	0.88 (13.618)	18.18 (16.949)	-17.30 (20.952)	0.4626
	95% CI for LS MEAN	(-38.55, 40.32)	(-31.83, 68.19)	(-79.45, 44.86)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Nausea and Vomiting

Subgroup by treatment interaction p-value	0.4646
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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain
Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.37 (3.238)	5.16 (4.558)	-6.53 (5.352)	0.2356
	95% CI for LS MEAN	(-8.03, 5.30)	(-4.27, 14.59)	(-17.63, 4.57)	
Week 4	LS Mean (SEM)	-2.12 (3.585)	0.16 (5.097)	-2.28 (6.018)	0.7069
	95% CI for LS MEAN	(-9.42, 5.17)	(-10.24, 10.56)	(-14.58, 10.01)	
Week 8	LS Mean (SEM)	-4.12 (3.779)	-4.84 (4.967)	0.72 (6.051)	0.9065
	95% CI for LS MEAN	(-11.92, 3.67)	(-15.17, 5.49)	(-11.87, 13.31)	
Week 12	LS Mean (SEM)	-6.35 (3.285)	5.16 (4.457)	-11.51 (5.300)	0.0409
	95% CI for LS MEAN	(-13.10, 0.39)	(-4.08, 14.40)	(-22.50, -0.52)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain
White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.67 (3.226)	0.93 (5.503)	-1.61 (6.370)	0.8024
	95% CI for LS MEAN	(-7.25, 5.91)	(-10.28, 12.15)	(-14.59, 11.38)	
Week 4	LS Mean (SEM)	-1.40 (1.966)	-3.00 (3.290)	1.60 (3.818)	0.6782
	95% CI for LS MEAN	(-5.40, 2.60)	(-9.69, 3.70)	(-6.17, 9.37)	
Week 8	LS Mean (SEM)	0.93 (4.686)	-5.59 (7.817)	6.52 (9.107)	0.4802
	95% CI for LS MEAN	(-8.69, 10.54)	(-21.62, 10.44)	(-12.16, 25.20)	
Week 12	LS Mean (SEM)	-0.51 (3.082)	-6.33 (5.040)	5.81 (5.897)	0.3332
	95% CI for LS MEAN	(-6.84, 5.81)	(-16.69, 4.03)	(-6.31, 17.93)	

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain
Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.49 (19.793)	21.16 (21.962)	-21.65 (22.262)	0.3494
	95% CI for LS MEAN	(-47.57, 46.59)	(-27.74, 70.05)	(-70.00, 26.70)	
Week 4	LS Mean (SEM)	6.65 (19.793)	36.52 (20.287)	-29.86 (20.721)	0.1791
	95% CI for LS MEAN	(-40.43, 53.73)	(-10.13, 83.16)	(-75.82, 16.09)	
Week 8	LS Mean (SEM)	25.16 (21.479)			
	95% CI for LS MEAN	(-23.86, 74.17)			
Week 12	LS Mean (SEM)	11.41 (19.793)	54.46 (21.895)	-43.05 (22.193)	0.0765
	95% CI for LS MEAN	(-35.67, 58.49)	(5.46, 103.46)	(-91.45, 5.36)	

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Pain

Subgroup by treatment interaction p-value	0.0132
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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea
Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-16.01 (5.233)	-4.15 (7.346)	-11.86 (8.461)	0.1737
	95% CI for LS MEAN	(-26.74, -5.27)	(-19.26, 10.96)	(-29.30, 5.59)	
Week 4	LS Mean (SEM)	-22.07 (5.148)	-0.82 (7.213)	-21.25 (8.292)	0.0161
	95% CI for LS MEAN	(-32.58, -11.55)	(-15.57, 13.93)	(-38.24, -4.26)	
Week 8	LS Mean (SEM)	-20.82 (5.274)	-17.48 (7.121)	-3.34 (8.304)	0.6911
	95% CI for LS MEAN	(-31.60, -10.04)	(-32.09, -2.88)	(-20.38, 13.71)	
Week 12	LS Mean (SEM)	-22.58 (5.187)	-14.15 (7.085)	-8.43 (8.206)	0.3147
	95% CI for LS MEAN	(-33.21, -11.95)	(-28.75, 0.45)	(-25.37, 8.51)	

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea
White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-12.55 (4.080)	-1.28 (7.071)	-11.26 (8.150)	0.1762
	95% CI for LS MEAN	(-20.86, -4.23)	(-15.66, 13.09)	(-27.84, 5.32)	
Week 4	LS Mean (SEM)	-19.99 (4.343)	-1.88 (7.312)	-18.11 (8.491)	0.0399
	95% CI for LS MEAN	(-28.81, -11.18)	(-16.72, 12.96)	(-35.35, -0.88)	
Week 8	LS Mean (SEM)	-12.96 (4.379)	3.89 (7.620)	-16.85 (8.774)	0.0648
	95% CI for LS MEAN	(-21.92, -4.00)	(-11.69, 19.47)	(-34.80, 1.10)	
Week 12	LS Mean (SEM)	-16.37 (3.617)	-5.21 (5.812)	-11.15 (6.842)	0.1137
	95% CI for LS MEAN	(-23.75, -8.98)	(-17.10, 6.67)	(-25.14, 2.83)	

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea
Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-31.72 (9.860)	11.78 (11.338)	-43.50 (13.879)	0.0050
	95% CI for LS MEAN	(-52.60, -10.84)	(-11.82, 35.38)	(-72.35, -14.65)	
Week 4	LS Mean (SEM)	-46.00 (9.860)	3.45 (11.338)	-49.45 (13.879)	0.0018
	95% CI for LS MEAN	(-66.88, -25.13)	(-20.16, 27.05)	(-78.30, -20.60)	
Week 8	LS Mean (SEM)	-47.61 (11.821)	3.85 (24.257)	-51.46 (25.628)	0.0567
	95% CI for LS MEAN	(-72.23, -22.99)	(-46.85, 54.55)	(-104.52, 1.60)	
Week 12	LS Mean (SEM)	-36.48 (9.860)	11.78 (11.338)	-48.26 (13.879)	0.0022
	95% CI for LS MEAN	(-57.36, -15.60)	(-11.82, 35.38)	(-77.11, -19.41)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Dyspnoea

Subgroup by treatment interaction p-value	0.0460
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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia
Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.68 (4.229)	6.23 (5.982)	-6.91 (6.927)	0.3286
	95% CI for LS MEAN	(-9.35, 8.00)	(-6.08, 18.54)	(-21.20, 7.39)	
Week 4	LS Mean (SEM)	-5.22 (4.574)	-0.44 (6.518)	-4.79 (7.597)	0.5340
	95% CI for LS MEAN	(-14.56, 4.12)	(-13.77, 12.90)	(-20.37, 10.80)	
Week 8	LS Mean (SEM)	-10.86 (4.610)	2.90 (6.258)	-13.75 (7.412)	0.0740
	95% CI for LS MEAN	(-20.25, -1.46)	(-9.90, 15.70)	(-28.93, 1.43)	
Week 12	LS Mean (SEM)	-7.53 (3.964)	6.23 (5.354)	-13.76 (6.234)	0.0362
	95% CI for LS MEAN	(-15.63, 0.57)	(-4.77, 17.23)	(-26.57, -0.96)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia
White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-6.81 (5.057)	-12.32 (8.558)	5.50 (9.912)	0.5827
	95% CI for LS MEAN	(-17.13, 3.50)	(-29.74, 5.11)	(-14.69, 25.70)	
Week 4	LS Mean (SEM)	-2.54 (5.224)	-12.59 (8.744)	10.05 (10.161)	0.3294
	95% CI for LS MEAN	(-13.15, 8.08)	(-30.36, 5.18)	(-10.60, 30.70)	
Week 8	LS Mean (SEM)	-9.58 (4.590)	-19.56 (7.964)	9.98 (9.143)	0.2866
	95% CI for LS MEAN	(-19.10, -0.06)	(-36.04, -3.09)	(-8.96, 28.92)	
Week 12	LS Mean (SEM)	-8.82 (5.406)	-5.92 (8.674)	-2.90 (10.194)	0.7782
	95% CI for LS MEAN	(-19.82, 2.18)	(-23.61, 11.76)	(-23.67, 17.87)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia
Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	0.33 (12.692)	20.50 (15.389)	-20.17 (18.845)	0.2983
	95% CI for LS MEAN	(-26.66, 27.32)	(-11.87, 52.87)	(-59.71, 19.37)	
Week 4	LS Mean (SEM)	-9.19 (12.692)	20.50 (15.389)	-29.70 (18.845)	0.1322
	95% CI for LS MEAN	(-36.18, 17.79)	(-11.87, 52.87)	(-69.24, 9.85)	
Week 8	LS Mean (SEM)	-15.54 (14.008)	18.31 (25.134)	-33.85 (27.884)	0.2395
	95% CI for LS MEAN	(-44.85, 13.78)	(-35.01, 71.64)	(-92.17, 24.48)	
Week 12	LS Mean (SEM)	-4.43 (12.692)	20.50 (15.389)	-24.93 (18.845)	0.2021
	95% CI for LS MEAN	(-31.42, 22.56)	(-11.87, 52.87)	(-64.48, 14.61)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Insomnia

Subgroup by treatment interaction p-value 0.0433

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss
Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-4.30 (2.622)	1.86 (3.741)	-6.16 (4.331)	0.1658
	95% CI for LS MEAN	(-9.67, 1.06)	(-5.80, 9.52)	(-15.04, 2.71)	
Week 4	LS Mean (SEM)	-4.30 (3.590)	-1.47 (5.217)	-2.83 (6.164)	0.6494
	95% CI for LS MEAN	(-11.61, 3.00)	(-12.11, 9.16)	(-15.41, 9.75)	
Week 8	LS Mean (SEM)	-0.48 (5.037)	-1.47 (6.927)	0.99 (8.439)	0.9073
	95% CI for LS MEAN	(-10.76, 9.80)	(-15.67, 12.72)	(-16.30, 18.28)	
Week 12	LS Mean (SEM)	-5.65 (4.017)	-1.47 (5.651)	-4.18 (6.793)	0.5441
	95% CI for LS MEAN	(-13.87, 2.57)	(-13.10, 10.15)	(-18.15, 9.80)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss
White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	3.09 (3.791)	2.64 (6.446)	0.45 (7.454)	0.9524
	95% CI for LS MEAN	(-4.65, 10.82)	(-10.49, 15.76)	(-14.74, 15.64)	
Week 4	LS Mean (SEM)	-5.58 (3.189)	2.75 (5.327)	-8.33 (6.185)	0.1878
	95% CI for LS MEAN	(-12.08, 0.92)	(-8.12, 13.61)	(-20.94, 4.28)	
Week 8	LS Mean (SEM)	4.60 (4.357)	3.68 (7.530)	0.92 (8.662)	0.9162
	95% CI for LS MEAN	(-4.40, 13.61)	(-11.84, 19.20)	(-16.96, 18.80)	
Week 12	LS Mean (SEM)	2.55 (5.428)	2.75 (8.907)	-0.19 (10.415)	0.9854
	95% CI for LS MEAN	(-8.60, 13.71)	(-15.61, 21.10)	(-21.65, 21.26)	

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss
Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	3.11 (8.411)	10.71 (10.026)	-7.60 (12.463)	0.5495
	95% CI for LS MEAN	(-14.84, 21.06)	(-10.35, 31.78)	(-33.76, 18.56)	
Week 4	LS Mean (SEM)	3.11 (8.411)	-14.29 (10.026)	17.40 (12.463)	0.1795
	95% CI for LS MEAN	(-14.84, 21.06)	(-35.35, 6.78)	(-8.76, 43.56)	
Week 8	LS Mean (SEM)	4.92 (8.915)	-5.08 (14.088)	10.01 (16.013)	0.5394
	95% CI for LS MEAN	(-13.91, 23.76)	(-34.94, 24.78)	(-23.47, 43.49)	
Week 12	LS Mean (SEM)	-1.65 (8.411)	10.71 (10.026)	-12.36 (12.463)	0.3342
	95% CI for LS MEAN	(-19.60, 16.30)	(-10.35, 31.78)	(-38.52, 13.80)	

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Appetite Loss

Subgroup by treatment interaction p-value	0.5734
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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation
Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-4.11 (4.466)	10.64 (6.397)	-14.75 (7.520)	0.0601
	95% CI for LS MEAN	(-13.24, 5.01)	(-2.46, 23.74)	(-30.18, 0.67)	
Week 4	LS Mean (SEM)	-2.60 (4.348)	10.64 (6.216)	-13.24 (7.296)	0.0813
	95% CI for LS MEAN	(-11.50, 6.30)	(-2.12, 23.39)	(-28.24, 1.77)	
Week 8	LS Mean (SEM)	-9.84 (3.509)	0.64 (4.640)	-10.48 (5.493)	0.0683
	95% CI for LS MEAN	(-17.07, -2.62)	(-8.93, 10.20)	(-21.82, 0.85)	
Week 12	LS Mean (SEM)	-3.35 (4.604)	7.31 (6.480)	-10.65 (7.672)	0.1761
	95% CI for LS MEAN	(-12.73, 6.04)	(-5.96, 20.57)	(-26.38, 5.07)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation
White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.45 (2.874)	-0.78 (4.933)	-1.68 (5.700)	0.7707
	95% CI for LS MEAN	(-8.32, 3.41)	(-10.82, 9.26)	(-13.28, 9.93)	
Week 4	LS Mean (SEM)	-1.51 (3.544)	2.14 (5.930)	-3.65 (6.897)	0.6000
	95% CI for LS MEAN	(-8.71, 5.69)	(-9.91, 14.19)	(-17.67, 10.36)	
Week 8	LS Mean (SEM)	-3.42 (4.076)	14.92 (6.974)	-18.35 (8.065)	0.0304
	95% CI for LS MEAN	(-11.77, 4.92)	(0.68, 29.17)	(-34.83, -1.86)	
Week 12	LS Mean (SEM)	-4.19 (3.455)	5.47 (5.638)	-9.67 (6.598)	0.1529
	95% CI for LS MEAN	(-11.23, 2.84)	(-6.03, 16.98)	(-23.12, 3.79)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation
Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-4.30 (16.132)	13.38 (18.011)	-17.68 (20.773)	0.4211
	95% CI for LS MEAN	(-42.64, 34.04)	(-28.94, 55.69)	(-66.12, 30.77)	
Week 4	LS Mean (SEM)	-9.06 (16.132)	21.71 (18.011)	-30.77 (20.773)	0.1792
	95% CI for LS MEAN	(-47.40, 29.28)	(-20.61, 64.03)	(-79.22, 17.68)	
Week 8	LS Mean (SEM)	-8.68 (16.994)	25.68 (24.378)	-34.35 (26.894)	0.2224
	95% CI for LS MEAN	(-47.95, 30.59)	(-26.26, 77.61)	(-92.07, 23.36)	
Week 12	LS Mean (SEM)	5.22 (16.132)	13.38 (18.011)	-8.15 (20.773)	0.7056
	95% CI for LS MEAN	(-33.12, 43.56)	(-28.94, 55.69)	(-56.60, 40.29)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Constipation

Subgroup by treatment interaction p-value	0.3812
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea
Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-2.74 (2.713)	5.75 (3.895)	-8.49 (4.577)	0.0745
	95% CI for LS MEAN	(-8.28, 2.80)	(-2.22, 13.73)	(-17.89, 0.90)	
Week 4	LS Mean (SEM)	-1.22 (2.693)	2.42 (3.866)	-3.65 (4.541)	0.4291
	95% CI for LS MEAN	(-6.72, 4.27)	(-5.49, 10.33)	(-12.96, 5.67)	
Week 8	LS Mean (SEM)	-0.55 (2.356)	-0.91 (3.158)	0.36 (3.737)	0.9240
	95% CI for LS MEAN	(-5.36, 4.26)	(-7.38, 5.56)	(-7.31, 8.03)	
Week 12	LS Mean (SEM)	0.44 (2.458)	-0.91 (3.346)	1.35 (3.958)	0.7354
	95% CI for LS MEAN	(-4.56, 5.44)	(-7.75, 5.93)	(-6.76, 9.46)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea
White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-3.61 (1.681)	-10.26 (2.975)	6.65 (3.470)	0.0635
	95% CI for LS MEAN	(-7.03, -0.18)	(-16.30, -4.22)	(-0.40, 13.69)	
Week 4	LS Mean (SEM)	-3.60 (2.127)	-10.29 (3.581)	6.69 (4.208)	0.1207
	95% CI for LS MEAN	(-7.92, 0.72)	(-17.56, -3.02)	(-1.85, 15.23)	
Week 8	LS Mean (SEM)	1.51 (3.968)	-2.49 (7.050)	4.00 (8.124)	0.6262
	95% CI for LS MEAN	(-6.60, 9.62)	(-16.87, 11.89)	(-12.57, 20.56)	
Week 12	LS Mean (SEM)	-3.96 (2.084)	-13.62 (3.352)	9.67 (3.991)	0.0220
	95% CI for LS MEAN	(-8.22, 0.31)	(-20.49, -6.75)	(1.50, 17.84)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea
Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	16.28 (11.613)	-7.43 (14.027)	23.71 (17.166)	0.1804
	95% CI for LS MEAN	(-7.94, 40.49)	(-36.49, 21.62)	(-11.79, 59.21)	
Week 4	LS Mean (SEM)	6.75 (11.613)	-7.43 (14.027)	14.19 (17.166)	0.4170
	95% CI for LS MEAN	(-17.46, 30.97)	(-36.49, 21.62)	(-21.31, 49.69)	
Week 8	LS Mean (SEM)	-3.82 (13.984)	-10.31 (28.329)	6.49 (29.900)	0.8298
	95% CI for LS MEAN	(-32.72, 25.08)	(-68.36, 47.75)	(-54.81, 67.79)	
Week 12	LS Mean (SEM)	1.99 (11.613)	9.23 (14.027)	-7.24 (17.166)	0.6770
	95% CI for LS MEAN	(-22.23, 26.21)	(-19.82, 38.29)	(-42.74, 28.26)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Diarrhea

Subgroup by treatment interaction p-value 0.1260

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties
Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.48 (3.510)	-1.69 (5.058)	0.20 (5.945)	0.9730
	95% CI for LS MEAN	(-8.70, 5.73)	(-12.12, 8.74)	(-12.09, 12.50)	
Week 4	LS Mean (SEM)	-4.51 (3.228)	-8.35 (4.625)	3.84 (5.408)	0.4835
	95% CI for LS MEAN	(-11.10, 2.07)	(-17.81, 1.10)	(-7.23, 14.91)	
Week 8	LS Mean (SEM)	-10.97 (4.135)	-1.69 (5.878)	-9.28 (7.025)	0.1977
	95% CI for LS MEAN	(-19.42, -2.52)	(-13.74, 10.36)	(-23.70, 5.14)	
Week 12	LS Mean (SEM)	-3.18 (4.479)	1.65 (6.475)	-4.83 (7.705)	0.5357
	95% CI for LS MEAN	(-12.30, 5.93)	(-11.56, 14.85)	(-20.56, 10.91)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties
White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.20 (3.590)	-5.57 (6.200)	5.38 (7.144)	0.4567
	95% CI for LS MEAN	(-7.49, 7.10)	(-18.14, 7.00)	(-9.12, 19.87)	
Week 4	LS Mean (SEM)	-1.39 (3.278)	-13.26 (5.493)	11.87 (6.377)	0.0711
	95% CI for LS MEAN	(-8.04, 5.27)	(-24.40, -2.11)	(-1.08, 24.81)	
Week 8	LS Mean (SEM)	-4.18 (3.059)	-3.12 (5.433)	-1.06 (6.212)	0.8660
	95% CI for LS MEAN	(-10.44, 2.08)	(-14.25, 8.00)	(-13.78, 11.66)	
Week 12	LS Mean (SEM)	0.47 (2.872)	-13.26 (4.693)	13.72 (5.479)	0.0173
	95% CI for LS MEAN	(-5.36, 6.30)	(-22.80, -3.71)	(2.58, 24.86)	

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Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:21

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.9
Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties
Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-12.32 (13.529)	33.11 (17.250)	-45.43 (20.161)	0.0560
	95% CI for LS MEAN	(-44.09, 19.45)	(-7.29, 73.51)	(-92.37, 1.50)	
Week 4	LS Mean (SEM)	-2.80 (13.529)	33.11 (17.250)	-35.91 (20.161)	0.1148
	95% CI for LS MEAN	(-34.57, 28.97)	(-7.29, 73.51)	(-82.84, 11.03)	
Week 8	LS Mean (SEM)	-5.76 (14.008)	31.70 (20.984)	-37.47 (23.585)	0.1395
	95% CI for LS MEAN	(-38.04, 26.52)	(-14.01, 77.42)	(-89.17, 14.23)	
Week 12	LS Mean (SEM)	-12.32 (13.529)	33.11 (17.250)	-45.43 (20.161)	0.0560
	95% CI for LS MEAN	(-44.09, 19.45)	(-7.29, 73.51)	(-92.37, 1.50)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:21

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.2.9

Change from Baseline in EORTC QLQ-C30 Scores Symptom Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Financial Difficulties

Subgroup by treatment interaction p-value	0.0018
---	--------

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Symptom scales include fatigue, nausea and vomiting, pain, dyspnoea, insomnia, appetite loss, constipation, diarrhoea, and financial difficulties.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:48:21

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.7 Allgemeiner Gesundheitszustand anhand der EQ-5D VAS

1.7.1 Rücklaufquote der EQ-5D VAS

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Table GERMANY.2.5.3.10
EQ-5D-3L Scores Compliance Rate During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Visit	Statistics	D->D (N=57)	P->D (N=26)
Baseline	Expected Patients Per Visit	57	26
	Patients Per Visit	57	26
	Patients with Completed VAS, n (%)	57 (100)	24 (92.3)
Week 2	Expected Patients Per Visit	57	26
	Patients Per Visit	57	26
	Patients with Completed VAS, n (%)	57 (100)	25 (96.2)
Week 4	Expected Patients Per Visit	57	25
	Patients Per Visit	57	25
	Patients with Completed VAS, n (%)	56 (98.2)	25 (100)
Week 8	Expected Patients Per Visit	57	25
	Patients Per Visit	57	25
	Patients with Completed VAS, n (%)	44 (77.2)	19 (76.0)
Week 12	Expected Patients Per Visit	55	25
	Patients Per Visit	54	25
	Patients with Completed VAS, n (%)	52 (94.5)	25 (100)
Week 14	Expected Patients Per Visit	55	24
	Patients Per Visit	55	24
	Patients with Completed VAS, n (%)	53 (96.4)	23 (95.8)

Percentages are based on the total number of expected patients in each group.

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.3.10
EQ-5D-3L Scores Compliance Rate During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Visit	Statistics	D->D (N=57)	P->D (N=26)
Week 16	Expected Patients Per Visit	55	24
	Patients Per Visit	55	24
	Patients with Completed VAS, n (%)	52 (94.5)	23 (95.8)
Week 20	Expected Patients Per Visit	55	24
	Patients Per Visit	53	24
	Patients with Completed VAS, n (%)	47 (85.5)	20 (83.3)
Week 24	Expected Patients Per Visit	55	24
	Patients Per Visit	55	24
	Patients with Completed VAS, n (%)	50 (90.9)	23 (95.8)
Week 40	Expected Patients Per Visit	46	22
	Patients Per Visit	46	22
	Patients with Completed VAS, n (%)	46 (100)	20 (90.9)
Week 56	Expected Patients Per Visit	36	18
	Patients Per Visit	36	18
	Patients with Completed VAS, n (%)	36 (100)	16 (88.9)
Week 72	Expected Patients Per Visit	21	12
	Patients Per Visit	21	12
	Patients with Completed VAS, n (%)	21 (100)	11 (91.7)

Percentages are based on the total number of expected patients in each group.

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.3.10
EQ-5D-3L Scores Compliance Rate During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Visit	Statistics	D->D (N=57)	P->D (N=26)
Week 88	Expected Patients Per Visit	14	8
	Patients Per Visit	14	8
	Patients with Completed VAS, n (%)	14 (100)	8 (100)
Week 104	Expected Patients Per Visit	4	2
	Patients Per Visit	4	2
	Patients with Completed VAS, n (%)	4 (100)	2 (100)

Percentages are based on the total number of expected patients in each group.

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.7 Allgemeiner Gesundheitszustand anhand der EQ-5D VAS

1.7.2 Veränderung der EQ-5D VAS gegenüber Baseline

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Table GERMANY.2.5.3.1
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	57		24		81	
Mean (SD)	59.1 (19.86)		60.9 (20.13)		59.7 (19.83)	
Median	60.0		69.0		65.0	
Min, Max	7, 98		9, 90		7, 98	
Week 2						
n	57	57	25	23		
Mean (SD)	68.2 (17.55)	9.1 (19.09)	63.8 (15.22)	4.1 (21.12)		
Median	70.0	10.0	60.0	0.0		
Min, Max	7, 100	-67, 63	30, 90	-25, 81		
Week 4						
n	56	56	25	24		
Mean (SD)	69.3 (17.40)	10.2 (17.40)	63.9 (18.71)	2.3 (24.27)		
Median	71.5	10.0	70.0	0.0		
Min, Max	30, 100	-24, 55	30, 90	-50, 81		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Data cutoff date of 31Mar2023

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Table GERMANY.2.5.3.1
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	44	44	19	18		
Mean (SD)	70.3 (20.15)	13.8 (18.59)	69.4 (17.51)	8.4 (25.50)		
Median	70.0	11.5	70.0	1.0		
Min, Max	8, 100	-25, 60	25, 90	-40, 81		
Week 12						
n	52	52	25	24		
Mean (SD)	73.1 (17.77)	14.3 (16.77)	66.0 (16.67)	5.0 (22.45)		
Median	72.0	10.0	70.0	0.0		
Min, Max	7, 100	-11, 65	30, 90	-35, 81		
Week 14						
n	53	53	23	22		
Mean (SD)	74.4 (14.61)	14.9 (17.23)	68.1 (19.82)	6.1 (26.17)		
Median	75.0	10.0	70.0	7.5		
Min, Max	45, 100	-10, 65	7, 90	-73, 81		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:45

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.3.1
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	52	52	23	22		
Mean (SD)	71.5 (18.15)	12.3 (19.29)	72.5 (15.36)	10.7 (18.81)		
Median	71.5	10.0	72.0	7.5		
Min, Max	15, 100	-35, 65	40, 95	-10, 81		
Week 20						
n	47	47	20	20		
Mean (SD)	72.0 (15.45)	10.8 (18.82)	72.7 (14.70)	12.9 (22.56)		
Median	75.0	6.0	76.5	6.5		
Min, Max	30, 100	-20, 68	40, 91	-20, 81		
Week 24						
n	50	50	23	22		
Mean (SD)	73.8 (16.70)	14.0 (19.08)	71.0 (18.28)	10.0 (22.01)		
Median	75.0	9.5	74.0	7.0		
Min, Max	20, 100	-20, 68	20, 91	-20, 82		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:45

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.3.1
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	46	46	20	20	66	66
Mean (SD)	70.7 (21.54)	12.1 (25.47)	66.0 (21.96)	5.2 (29.07)	69.3 (21.61)	10.0 (26.58)
Median	75.0	10.0	70.0	9.0	70.0	10.0
Min, Max	7, 100	-67, 70	7, 98	-68, 81	7, 100	-68, 81
Week 56						
n	36	36	16	15	52	51
Mean (SD)	69.2 (21.27)	11.6 (27.46)	70.1 (16.32)	8.3 (24.00)	69.5 (19.72)	10.6 (26.30)
Median	70.0	10.0	72.5	0.0	70.0	10.0
Min, Max	0, 100	-98, 65	30, 90	-14, 81	0, 100	-98, 81
Week 72						
n	21	21	11	10	32	31
Mean (SD)	67.7 (24.56)	8.2 (27.78)	64.0 (24.15)	-7.4 (25.06)	66.4 (24.09)	3.2 (27.53)
Median	72.0	5.0	73.0	-3.5	72.5	2.0
Min, Max	8, 100	-62, 70	6, 80	-62, 40	6, 100	-62, 70

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:45

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.3.1
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	77.7 (13.56)	12.9 (16.56)	72.5 (8.45)	5.9 (15.08)	75.8 (12.01)	10.3 (16.04)
Median	80.0	10.0	75.0	5.0	80.0	10.0
Min, Max	50, 100	-20, 45	60, 80	-15, 30	50, 100	-20, 45
Week 104						
n	4	4	2	2	6	6
Mean (SD)	82.5 (17.56)	11.8 (10.44)	72.5 (10.61)	10.0 (28.28)	79.2 (15.30)	11.2 (15.04)
Median	82.5	10.0	72.5	10.0	75.0	10.0
Min, Max	65, 100	2, 25	65, 80	-10, 30	65, 100	-10, 30

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:45

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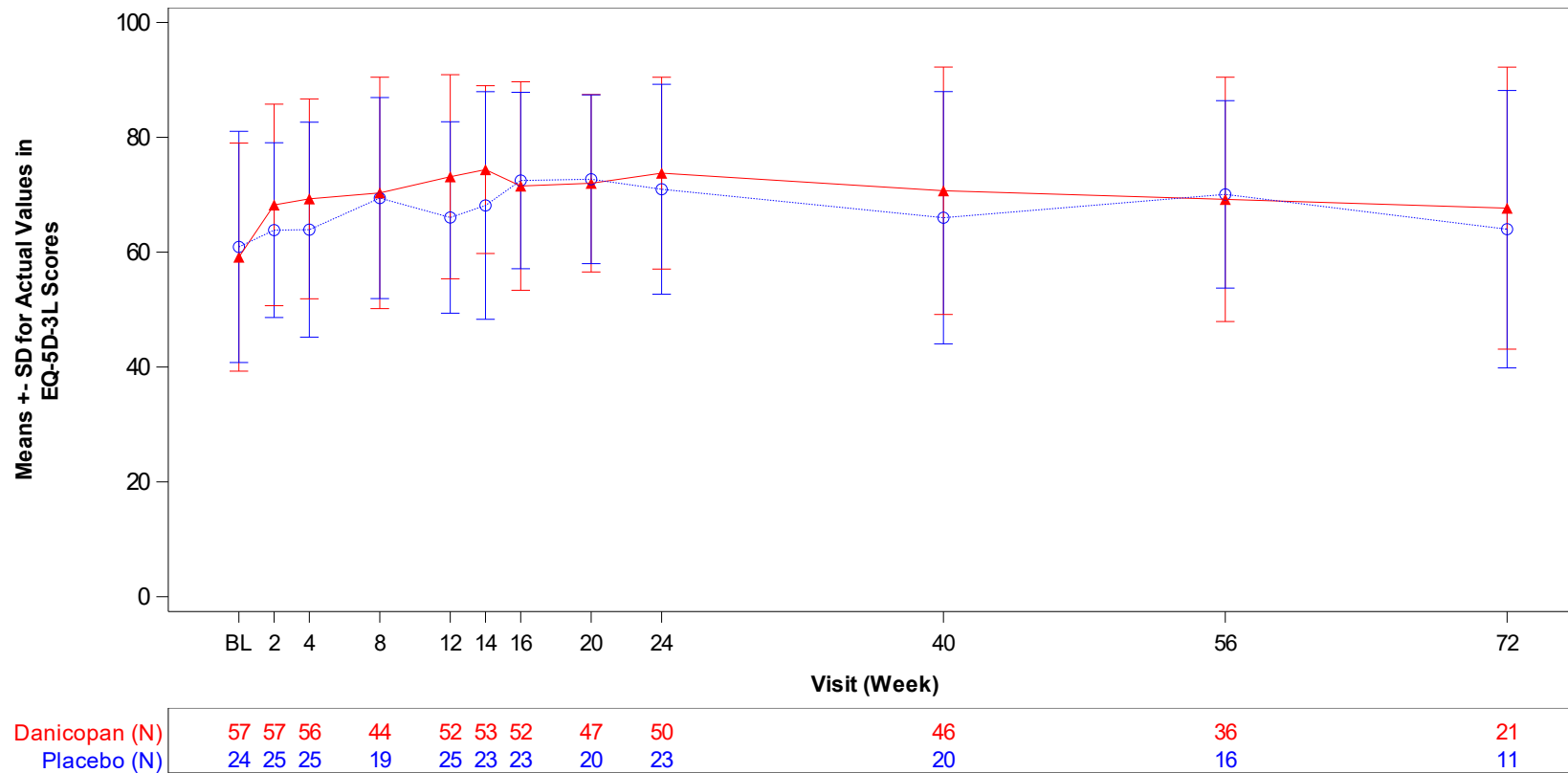
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Figure GERMANY.2.2.9.3
Plots of Mean \pm SD for Actual Values in EQ-5D-3L Scores During 72 Weeks
Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Source: ADaM.ADQS

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Data cutoff date of 31Mar2023

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Table GERMANY.2.5.1.1
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	8.86 (2.109)	4.30 (3.278)	4.56 (3.805)	0.2347
	95% CI for LS MEAN	(4.66, 13.06)	(-2.22, 10.82)	(-3.02, 12.13)	
Week 4	LS Mean (SEM)	10.40 (2.203)	3.10 (3.361)	7.30 (3.934)	0.0677
	95% CI for LS MEAN	(6.01, 14.78)	(-3.59, 9.80)	(-0.54, 15.14)	
Week 8	LS Mean (SEM)	11.05 (2.473)	6.40 (3.808)	4.65 (4.465)	0.3012
	95% CI for LS MEAN	(6.13, 15.98)	(-1.18, 13.99)	(-4.25, 13.55)	
Week 12	LS Mean (SEM)	13.16 (2.139)	5.73 (3.216)	7.44 (3.775)	0.0526
	95% CI for LS MEAN	(8.90, 17.42)	(-0.68, 12.13)	(-0.09, 14.96)	

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). An Unstructured covariance structure was used.

P-value is from difference of LSM from MMRM model.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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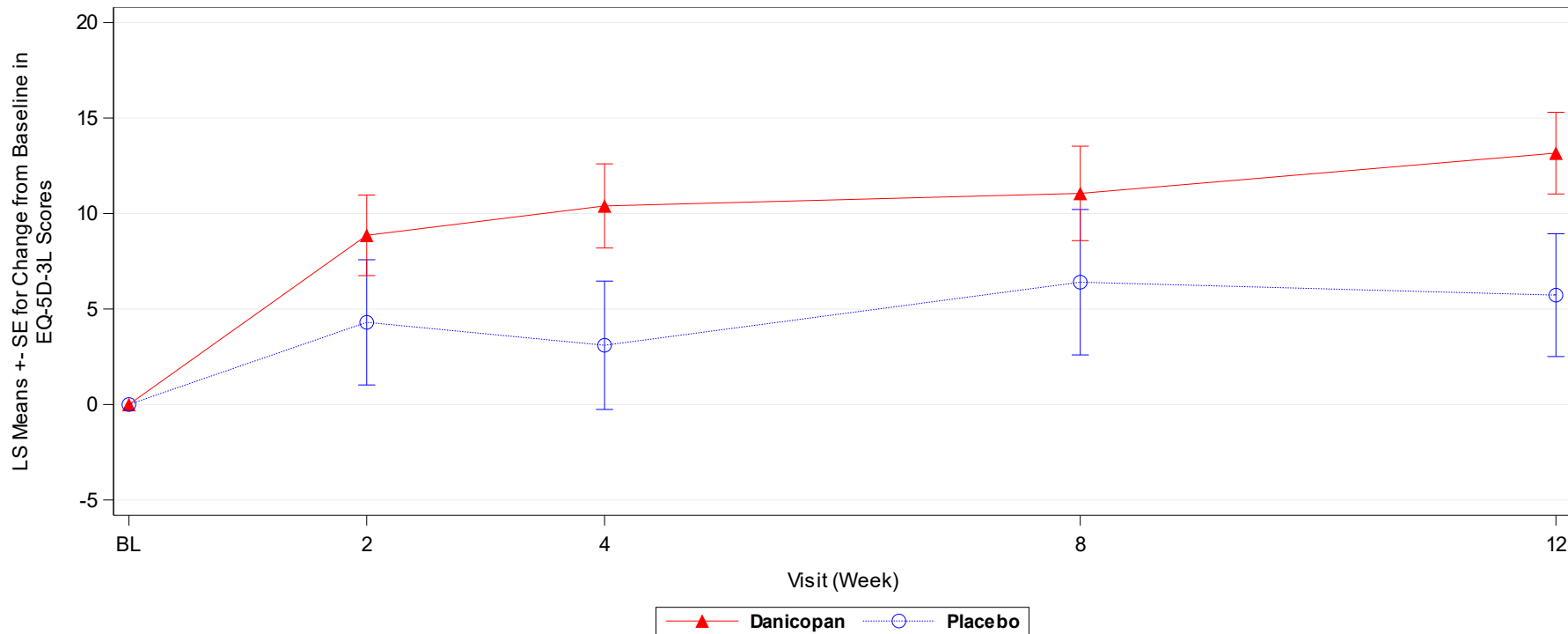
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Figure GERMANY.2.2.9.1
Plots of LS Means \pm SE for Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).
EQ-5D-3L is Three-level EuroQoL 5 dimensions.
Visual Analogue Scale (VAS) Score is reported.
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Data cutoff date of 31Mar2023
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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.7 Allgemeiner Gesundheitszustand anhand der EQ-5D VAS

1.7.2 Veränderung der EQ-5D VAS gegenüber Baseline

1.7.2.1 Subgruppenanalysen – Veränderung der EQ-5D VAS gegenüber Baseline

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Table GERMANY.2.5.3.2
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean (SD)	61.7 (19.85)		63.8 (22.63)		62.3 (20.31)	
Median	70.0		70.0		70.0	
Min, Max	25, 95		9, 80		9, 95	
Week 2						
n	23	23	8	8		
Mean (SD)	69.1 (19.17)	7.3 (22.29)	75.0 (11.65)	13.3 (28.99)		
Median	70.0	10.0	75.0	5.0		
Min, Max	7, 95	-67, 55	60, 90	-10, 81		
Week 4						
n	23	23	9	9		
Mean (SD)	70.7 (16.72)	9.0 (18.37)	72.3 (11.74)	8.6 (27.90)		
Median	75.0	10.0	75.0	0.0		
Min, Max	30, 95	-24, 55	50, 90	-10, 81		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.
Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.2
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	19	19	7	7		
Mean (SD)	75.1 (15.36)	12.9 (16.72)	81.7 (8.60)	18.3 (28.72)		
Median	75.0	11.0	80.0	10.0		
Min, Max	50, 97	-10, 60	70, 90	0, 81		
Week 12						
n	22	22	9	9		
Mean (SD)	73.5 (14.55)	12.1 (17.22)	72.3 (16.05)	8.6 (29.09)		
Median	72.5	9.0	76.0	0.0		
Min, Max	40, 97	-10, 65	35, 90	-15, 81		
Week 14						
n	22	22	9	9		
Mean (SD)	75.9 (14.11)	14.5 (16.36)	69.8 (26.20)	6.0 (39.15)		
Median	75.0	10.5	80.0	5.0		
Min, Max	50, 96	-10, 65	7, 90	-73, 81		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.3.2
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	22	22	8	8		
Mean (SD)	75.2 (14.34)	13.8 (17.34)	80.3 (9.36)	17.3 (27.15)		
Median	75.0	10.0	80.0	7.5		
Min, Max	45, 95	-10, 65	65, 90	0, 81		
Week 20						
n	20	20	9	9		
Mean (SD)	76.9 (13.12)	12.4 (19.70)	80.3 (11.01)	16.6 (28.26)		
Median	75.5	7.0	80.0	10.0		
Min, Max	50, 98	-10, 65	60, 91	-20, 81		
Week 24						
n	21	21	9	9		
Mean (SD)	74.4 (18.39)	11.5 (20.00)	81.3 (10.43)	17.6 (27.97)		
Median	75.0	9.0	82.0	12.0		
Min, Max	20, 100	-20, 68	60, 91	-20, 82		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.3.2
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	8	8	29	29
Mean (SD)	68.2 (24.82)	7.0 (30.96)	77.6 (9.10)	15.9 (28.07)	70.8 (21.88)	9.5 (29.96)
Median	73.0	10.0	75.0	11.0	73.0	10.0
Min, Max	7, 100	-67, 70	69, 90	-10, 81	7, 100	-67, 81
Week 56						
n	17	17	5	5	22	22
Mean (SD)	75.7 (16.05)	14.5 (21.33)	83.0 (6.71)	25.2 (33.21)	77.4 (14.65)	16.9 (24.04)
Median	80.0	11.0	80.0	10.0	80.0	10.5
Min, Max	40, 100	-20, 65	75, 90	0, 81	40, 100	-20, 81
Week 72						
n	8	8	2	2	10	10
Mean (SD)	60.0 (27.77)	0.6 (39.68)	75.0 (7.07)	-5.0 (7.07)	63.0 (25.40)	-0.5 (35.15)
Median	70.0	3.5	75.0	-5.0	70.0	1.0
Min, Max	8, 95	-62, 70	70, 80	-10, 0	8, 95	-62, 70

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.3.2
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	2	2	8	8
Mean (SD)	74.2 (12.01)	7.5 (15.08)	75.0 (7.07)	-5.0 (7.07)	74.4 (10.50)	4.4 (14.25)
Median	80.0	10.0	75.0	-5.0	80.0	7.5
Min, Max	50, 80	-20, 25	70, 80	-10, 0	50, 80	-20, 25
Week 104						
n	1	1	0	0	1	1
Mean (SD)	70.0 (NA)	5.0 (NA)			70.0 (NA)	5.0 (NA)
Median	70.0	5.0			70.0	5.0
Min, Max	70, 70	5, 5			70, 70	5, 5

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.3.2
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		15		49	
Mean (SD)	57.4 (19.97)		59.2 (19.09)		57.9 (19.53)	
Median	60.0		60.0		60.0	
Min, Max	7, 98		30, 90		7, 98	
Week 2						
n	34	34	17	15		
Mean (SD)	67.6 (16.63)	10.3 (16.85)	58.6 (14.01)	-0.8 (14.39)		
Median	70.0	10.0	60.0	0.0		
Min, Max	40, 100	-16, 63	30, 85	-25, 30		
Week 4						
n	33	33	16	15		
Mean (SD)	68.3 (18.05)	11.0 (16.92)	59.2 (20.51)	-1.4 (21.98)		
Median	71.0	10.0	62.5	-3.0		
Min, Max	30, 100	-20, 49	30, 90	-50, 50		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.
Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.3.2
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	25	25	12	11		
Mean (SD)	66.7 (22.77)	14.4 (20.22)	62.3 (17.58)	2.2 (22.35)		
Median	70.0	16.0	65.0	0.0		
Min, Max	8, 100	-25, 45	25, 85	-40, 40		
Week 12						
n	30	30	16	15		
Mean (SD)	72.9 (20.05)	15.8 (16.54)	62.5 (16.43)	2.8 (18.20)		
Median	71.0	10.0	65.0	0.0		
Min, Max	7, 100	-11, 48	30, 90	-35, 40		
Week 14						
n	31	31	14	13		
Mean (SD)	73.3 (15.09)	15.2 (18.08)	67.1 (15.47)	6.2 (13.28)		
Median	76.0	10.0	67.5	10.0		
Min, Max	45, 100	-10, 58	40, 90	-10, 40		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.
Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.2
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	30	30	15	14		
Mean (SD)	68.8 (20.33)	11.1 (20.81)	68.3 (16.56)	6.9 (11.53)		
Median	70.0	8.5	70.0	7.0		
Min, Max	15, 100	-35, 48	40, 95	-10, 41		
Week 20						
n	27	27	11	11		
Mean (SD)	68.4 (16.27)	9.6 (18.42)	66.5 (14.77)	9.8 (17.48)		
Median	70.0	5.0	70.0	5.0		
Min, Max	30, 100	-20, 68	40, 81	-10, 51		
Week 24						
n	29	29	14	13		
Mean (SD)	73.3 (15.69)	15.9 (18.52)	64.3 (19.39)	4.8 (15.92)		
Median	75.0	11.0	67.5	0.0		
Min, Max	30, 100	-15, 58	20, 90	-20, 41		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.3.2
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	25	25	12	12	37	37
Mean (SD)	72.8 (18.64)	16.4 (19.41)	58.3 (24.83)	-2.0 (28.61)	68.1 (21.62)	10.4 (24.03)
Median	80.0	10.0	62.0	6.5	70.0	10.0
Min, Max	35, 100	-15, 50	7, 98	-68, 30	7, 100	-68, 50
Week 56						
n	19	19	11	10	30	29
Mean (SD)	63.4 (23.98)	9.1 (32.37)	64.2 (16.12)	-0.2 (12.91)	63.7 (21.14)	5.9 (27.34)
Median	70.0	9.0	65.0	0.0	67.5	0.0
Min, Max	0, 97	-98, 53	30, 87	-14, 30	0, 97	-98, 53
Week 72						
n	13	13	9	8	22	21
Mean (SD)	72.4 (22.18)	12.9 (17.48)	61.6 (26.19)	-8.0 (28.25)	68.0 (23.92)	5.0 (23.90)
Median	75.0	5.0	73.0	-3.5	75.0	2.0
Min, Max	35, 100	-10, 46	6, 80	-62, 40	6, 100	-62, 46

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.3.2
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	8	8	6	6	14	14
Mean (SD)	80.4 (14.82)	16.9 (17.42)	71.7 (9.31)	9.5 (15.67)	76.6 (13.10)	13.7 (16.50)
Median	82.5	15.0	75.0	11.0	77.5	11.0
Min, Max	60, 100	-7, 45	60, 80	-15, 30	60, 100	-15, 45
Week 104						
n	3	3	2	2	5	5
Mean (SD)	86.7 (18.93)	14.0 (11.53)	72.5 (10.61)	10.0 (28.28)	81.0 (16.36)	12.4 (16.47)
Median	95.0	15.0	72.5	10.0	80.0	15.0
Min, Max	65, 100	2, 25	65, 80	-10, 30	65, 100	-10, 30

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.3
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and
Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	41		18		59	
Mean (SD)	59.1 (21.63)		57.9 (21.77)		58.7 (21.50)	
Median	65.0		62.5		65.0	
Min, Max	7, 98		9, 90		7, 98	
Week 2						
n	41	41	20	18		
Mean (SD)	70.1 (18.79)	11.0 (20.17)	62.6 (15.34)	4.9 (23.30)		
Median	71.0	10.0	60.0	0.0		
Min, Max	7, 100	-67, 63	30, 90	-25, 81		
Week 4						
n	40	40	19	18		
Mean (SD)	69.5 (19.08)	10.4 (18.26)	63.5 (20.86)	4.7 (27.63)		
Median	75.0	10.0	70.0	0.0		
Min, Max	30, 100	-24, 55	30, 90	-50, 81		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.3
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and
Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	31	31	14	13		
Mean (SD)	72.5 (21.26)	16.7 (17.65)	67.8 (19.32)	9.4 (29.28)		
Median	75.0	15.0	71.0	2.0		
Min, Max	8, 100	-10, 60	25, 90	-40, 81		
Week 12						
n	39	39	19	18		
Mean (SD)	74.0 (18.69)	14.4 (16.35)	65.5 (17.71)	7.4 (25.30)		
Median	73.0	10.0	70.0	0.0		
Min, Max	7, 100	-11, 65	30, 90	-35, 81		
Week 14						
n	39	39	17	16		
Mean (SD)	75.8 (15.45)	16.3 (18.07)	70.5 (16.54)	11.7 (22.24)		
Median	76.0	10.0	70.0	10.0		
Min, Max	45, 100	-10, 65	40, 90	-10, 81		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.3
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and
Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	38	38	18	17		
Mean (SD)	72.8 (20.46)	13.6 (19.80)	72.3 (16.99)	12.9 (20.87)		
Median	75.0	10.0	72.0	10.0		
Min, Max	15, 100	-35, 65	40, 95	-10, 81		
Week 20						
n	34	34	14	14		
Mean (SD)	72.5 (16.93)	11.1 (18.77)	72.0 (16.46)	16.5 (24.58)		
Median	75.0	6.0	77.5	7.5		
Min, Max	30, 100	-20, 68	40, 90	-10, 81		
Week 24						
n	37	37	17	16		
Mean (SD)	74.9 (18.00)	14.2 (19.60)	69.8 (20.17)	12.1 (24.44)		
Median	75.0	9.0	71.0	6.0		
Min, Max	20, 100	-15, 68	20, 91	-20, 82		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.3
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	33	33	15	15	48	48
Mean (SD)	71.2 (23.45)	11.9 (27.56)	63.9 (24.75)	5.4 (33.49)	68.9 (23.85)	9.9 (29.34)
Median	75.0	10.0	69.0	8.0	70.0	9.5
Min, Max	7, 100	-67, 70	7, 98	-68, 81	7, 100	-68, 81
Week 56						
n	25	25	14	13	39	38
Mean (SD)	70.2 (23.53)	12.5 (30.04)	70.1 (17.09)	10.3 (25.17)	70.1 (21.20)	11.7 (28.15)
Median	79.0	11.0	72.5	2.0	76.0	10.0
Min, Max	0, 100	-98, 60	30, 90	-14, 81	0, 100	-98, 81
Week 72						
n	15	15	9	8	24	23
Mean (SD)	67.4 (28.03)	8.5 (32.63)	67.1 (23.86)	-5.5 (27.92)	67.3 (26.00)	3.7 (31.18)
Median	75.0	5.0	75.0	-1.0	75.0	0.0
Min, Max	8, 100	-62, 70	6, 80	-62, 40	6, 100	-62, 70

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Table GERMANY.2.5.3.3
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and
Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	8	8	6	6	14	14
Mean (SD)	80.0 (15.35)	12.1 (19.28)	75.0 (7.75)	7.8 (16.13)	77.9 (12.51)	10.3 (17.47)
Median	80.0	10.0	77.5	6.0	80.0	10.0
Min, Max	50, 100	-20, 45	60, 80	-15, 30	50, 100	-20, 45
Week 104						
n	3	3	2	2	5	5
Mean (SD)	88.3 (16.07)	7.3 (6.81)	72.5 (10.61)	10.0 (28.28)	82.0 (15.25)	8.4 (15.01)
Median	95.0	5.0	72.5	10.0	80.0	5.0
Min, Max	70, 100	2, 15	65, 80	-10, 30	65, 100	-10, 30

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.3
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean (SD)	59.3 (14.98)		70.0 (10.95)		62.2 (14.58)	
Median	60.0		70.0		65.0	
Min, Max	25, 90		50, 80		25, 90	
Week 2						
n	16	16	5	5		
Mean (SD)	63.4 (13.20)	4.1 (15.47)	69.0 (15.17)	1.0 (11.40)		
Median	61.0	0.0	70.0	0.0		
Min, Max	40, 95	-20, 37	50, 90	-10, 20		
Week 4						
n	16	16	6	6		
Mean (SD)	68.8 (12.79)	9.5 (15.56)	65.2 (10.59)	-4.8 (5.67)		
Median	70.0	10.0	65.0	-5.0		
Min, Max	50, 90	-20, 39	50, 80	-10, 1		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.3
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	13	13	5	5		
Mean (SD)	65.1 (16.81)	6.7 (19.57)	74.0 (11.40)	6.0 (13.42)		
Median	70.0	0.0	70.0	0.0		
Min, Max	25, 90	-25, 40	60, 90	-10, 20		
Week 12						
n	13	13	6	6		
Mean (SD)	70.6 (15.05)	13.8 (18.66)	67.7 (14.17)	-2.3 (7.53)		
Median	70.0	19.0	70.0	-2.0		
Min, Max	50, 95	-10, 37	40, 80	-10, 10		
Week 14						
n	14	14	6	6		
Mean (SD)	70.4 (11.51)	11.1 (14.55)	61.3 (27.89)	-8.7 (32.09)		
Median	70.0	14.5	70.0	2.5		
Min, Max	50, 85	-10, 35	7, 81	-73, 11		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.3
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and
Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	14	14	5	5		
Mean (SD)	68.0 (9.14)	8.8 (18.03)	73.0 (8.37)	3.0 (4.47)		
Median	70.0	3.5	75.0	0.0		
Min, Max	50, 80	-25, 42	60, 80	0, 10		
Week 20						
n	13	13	6	6		
Mean (SD)	70.7 (11.15)	10.0 (19.69)	74.3 (10.56)	4.3 (15.47)		
Median	71.0	0.0	74.0	3.5		
Min, Max	50, 84	-10, 59	60, 91	-20, 21		
Week 24						
n	13	13	6	6		
Mean (SD)	70.5 (12.29)	13.6 (18.25)	74.3 (12.23)	4.3 (13.76)		
Median	75.0	18.0	77.0	7.0		
Min, Max	40, 85	-20, 50	60, 90	-20, 20		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

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Table GERMANY.2.5.3.3
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	13	13	5	5	18	18
Mean (SD)	69.3 (16.48)	12.5 (20.20)	72.4 (8.88)	4.4 (9.32)	70.2 (14.57)	10.3 (17.96)
Median	80.0	10.0	70.0	10.0	75.0	10.0
Min, Max	40, 85	-20, 59	60, 82	-10, 12	40, 85	-20, 59
Week 56						
n	11	11	2	2	13	13
Mean (SD)	67.0 (15.72)	9.7 (21.63)	70.0 (14.14)	-5.0 (7.07)	67.5 (14.96)	7.5 (20.61)
Median	70.0	0.0	70.0	-5.0	70.0	0.0
Min, Max	40, 90	-20, 65	60, 80	-10, 0	40, 90	-20, 65
Week 72						
n	6	6	2	2	8	8
Mean (SD)	68.3 (14.57)	7.5 (10.25)	50.0 (28.28)	-15.0 (7.07)	63.8 (18.38)	1.9 (13.81)
Median	71.0	5.0	50.0	-15.0	70.0	3.5
Min, Max	45, 88	0, 28	30, 70	-20, -10	30, 88	-20, 28

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.3.3
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and
Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	2	2	8	8
Mean (SD)	74.7 (11.34)	13.8 (13.79)	65.0 (7.07)	0.0 (14.14)	72.3 (10.91)	10.4 (14.33)
Median	77.5	15.0	65.0	0.0	72.5	10.0
Min, Max	60, 90	-7, 30	60, 70	-10, 10	60, 90	-10, 30
Week 104						
n	1	1	0	0	1	1
Mean (SD)	65.0 (NA)	25.0 (NA)			65.0 (NA)	25.0 (NA)
Median	65.0	25.0			65.0	25.0
Min, Max	65, 65	25, 25			65, 65	25, 25

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.3.4
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	25		12		37	
Mean (SD)	58.7 (18.24)		64.4 (15.09)		60.5 (17.28)	
Median	60.0		66.5		60.0	
Min, Max	25, 95		40, 90		25, 95	
Week 2						
n	25	25	13	12		
Mean (SD)	68.5 (19.96)	9.8 (21.88)	60.0 (13.54)	-4.4 (12.12)		
Median	70.0	10.0	60.0	0.0		
Min, Max	7, 100	-67, 40	30, 85	-25, 10		
Week 4						
n	24	24	13	12		
Mean (SD)	65.3 (18.21)	6.7 (17.26)	59.8 (17.12)	-6.3 (16.75)		
Median	66.0	6.0	65.0	-1.5		
Min, Max	30, 95	-24, 39	30, 80	-50, 10		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.
Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.4
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	18	18	8	7		
Mean (SD)	70.4 (19.82)	13.3 (19.03)	68.0 (11.75)	-2.0 (19.67)		
Median	70.0	17.5	70.0	0.0		
Min, Max	25, 100	-25, 40	50, 85	-40, 20		
Week 12						
n	23	23	13	12		
Mean (SD)	73.7 (15.76)	16.0 (15.66)	61.9 (18.20)	-3.2 (15.62)		
Median	70.0	10.0	65.0	0.0		
Min, Max	40, 100	-8, 40	30, 90	-35, 20		
Week 14						
n	24	24	12	11		
Mean (SD)	72.2 (14.52)	13.1 (14.91)	67.8 (15.37)	2.8 (9.66)		
Median	72.5	15.5	67.5	5.0		
Min, Max	45, 100	-10, 40	40, 90	-10, 20		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.4
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	23	23	13	12		
Mean (SD)	68.9 (18.05)	10.3 (19.94)	70.8 (16.07)	5.7 (8.99)		
Median	70.0	5.0	72.0	3.0		
Min, Max	30, 100	-25, 48	40, 95	-10, 25		
Week 20						
n	20	20	9	9		
Mean (SD)	72.4 (14.66)	8.8 (18.64)	69.1 (15.52)	8.2 (12.59)		
Median	70.0	5.0	70.0	5.0		
Min, Max	30, 100	-10, 59	40, 90	-10, 25		
Week 24						
n	21	21	12	11		
Mean (SD)	71.3 (18.54)	11.7 (18.83)	70.3 (16.97)	7.4 (11.88)		
Median	75.0	5.0	72.5	2.0		
Min, Max	20, 100	-15, 50	30, 90	-10, 25		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.4
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	11	11	29	29
Mean (SD)	62.3 (25.79)	6.9 (32.20)	64.4 (22.89)	1.8 (25.29)	63.1 (24.33)	5.0 (29.40)
Median	67.5	10.0	69.0	8.0	69.0	10.0
Min, Max	7, 100	-67, 59	7, 98	-68, 30	7, 100	-68, 59
Week 56						
n	13	13	10	9	23	22
Mean (SD)	63.2 (19.28)	9.8 (22.76)	69.1 (11.96)	5.9 (15.46)	65.8 (16.43)	8.2 (19.77)
Median	70.0	0.0	70.0	2.0	70.0	1.0
Min, Max	40, 97	-20, 65	50, 87	-14, 30	40, 97	-20, 65
Week 72						
n	8	8	7	6	15	14
Mean (SD)	58.0 (27.69)	-0.8 (29.65)	56.3 (27.73)	-17.3 (23.22)	57.2 (26.72)	-7.9 (27.45)
Median	65.0	5.0	70.0	-10.0	70.0	-1.0
Min, Max	8, 96	-62, 46	6, 80	-62, 0	6, 96	-62, 46

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.4
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	5	5	11	11
Mean (SD)	76.3 (12.91)	17.2 (17.50)	74.0 (8.22)	7.4 (16.55)	75.3 (10.57)	12.7 (16.99)
Median	80.0	15.0	75.0	10.0	80.0	10.0
Min, Max	60, 95	-7, 45	60, 80	-15, 30	60, 95	-15, 45
Week 104						
n	1	1	2	2	3	3
Mean (SD)	65.0 (NA)	25.0 (NA)	72.5 (10.61)	10.0 (28.28)	70.0 (8.66)	15.0 (21.79)
Median	65.0	25.0	72.5	10.0	65.0	25.0
Min, Max	65, 65	25, 25	65, 80	-10, 30	65, 80	-10, 30

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Table GERMANY.2.5.3.4
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	59.5 (21.33)		57.4 (24.35)		58.9 (21.92)	
Median	62.5		70.0		65.0	
Min, Max	7, 98		9, 80		7, 98	
Week 2						
n	32	32	12	11		
Mean (SD)	68.0 (15.74)	8.5 (16.94)	68.0 (16.40)	13.4 (25.26)		
Median	70.0	5.0	70.0	6.0		
Min, Max	40, 100	-20, 63	36, 90	-10, 81		
Week 4						
n	32	32	12	12		
Mean (SD)	72.3 (16.42)	12.8 (17.30)	68.4 (20.05)	11.0 (28.06)		
Median	75.0	10.0	70.5	0.0		
Min, Max	30, 100	-20, 55	30, 90	-10, 81		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.4
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	26	26	11	11		
Mean (SD)	70.3 (20.76)	14.1 (18.66)	70.5 (21.27)	15.1 (27.35)		
Median	72.0	11.0	80.0	10.0		
Min, Max	8, 100	-10, 60	25, 90	-15, 81		
Week 12						
n	29	29	12	12		
Mean (SD)	72.7 (19.49)	12.9 (17.75)	70.5 (14.25)	13.1 (25.80)		
Median	74.0	10.0	73.0	0.0		
Min, Max	7, 100	-11, 65	40, 90	-10, 81		
Week 14						
n	29	29	11	11		
Mean (SD)	76.2 (14.68)	16.4 (19.06)	68.5 (24.57)	9.5 (36.34)		
Median	80.0	10.0	75.0	10.0		
Min, Max	45, 100	-10, 65	7, 90	-73, 81		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.
Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.4
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	29	29	10	10		
Mean (SD)	73.6 (18.29)	13.8 (18.96)	74.6 (14.96)	16.7 (25.56)		
Median	75.0	10.0	77.5	10.0		
Min, Max	15, 100	-35, 65	50, 90	0, 81		
Week 20						
n	27	27	11	11		
Mean (SD)	71.7 (16.28)	12.2 (19.17)	75.6 (14.02)	16.6 (28.38)		
Median	75.0	9.0	78.0	8.0		
Min, Max	45, 97	-20, 68	45, 91	-20, 81		
Week 24						
n	29	29	11	11		
Mean (SD)	75.6 (15.33)	15.8 (19.40)	71.6 (20.43)	12.6 (29.34)		
Median	77.0	10.0	74.0	10.0		
Min, Max	40, 100	-20, 68	20, 91	-20, 82		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.4
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	28	28	9	9	37	37
Mean (SD)	76.1 (16.68)	15.4 (19.99)	68.0 (21.96)	9.2 (34.26)	74.1 (18.12)	13.9 (23.83)
Median	79.5	10.0	70.0	10.0	79.0	10.0
Min, Max	35, 100	-20, 70	30, 90	-50, 81	30, 100	-50, 81
Week 56						
n	23	23	6	6	29	29
Mean (SD)	72.6 (22.01)	12.7 (30.23)	71.7 (23.17)	11.8 (34.70)	72.4 (21.83)	12.5 (30.55)
Median	80.0	12.0	80.0	0.0	80.0	11.0
Min, Max	0, 100	-98, 60	30, 90	-10, 81	0, 100	-98, 81
Week 72						
n	13	13	4	4	17	17
Mean (SD)	73.6 (21.38)	13.8 (26.20)	77.5 (5.00)	7.5 (22.17)	74.5 (18.72)	12.3 (24.79)
Median	75.0	10.0	80.0	0.0	80.0	5.0
Min, Max	30, 100	-40, 70	70, 80	-10, 40	30, 100	-40, 70

EQ-5D-3L is Three-level EuroQoL 5 dimensions.
Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.4
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	8	8	3	3	11	11
Mean (SD)	78.8 (14.82)	9.6 (16.20)	70.0 (10.00)	3.3 (15.28)	76.4 (13.80)	7.9 (15.46)
Median	80.0	7.5	70.0	0.0	80.0	5.0
Min, Max	50, 100	-20, 30	60, 80	-10, 20	50, 100	-20, 30
Week 104						
n	3	3	0	0	3	3
Mean (SD)	88.3 (16.07)	7.3 (6.81)			88.3 (16.07)	7.3 (6.81)
Median	95.0	5.0			95.0	5.0
Min, Max	70, 100	2, 15			70, 100	2, 15

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.5

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Visual Analogue Scale (VAS) Score

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		9		33	
Mean (SD)	59.3 (19.70)		64.2 (15.22)		60.6 (18.49)	
Median	60.0		68.0		60.0	
Min, Max	30, 98		40, 80		30, 98	
Week 2						
n	24	24	9	8		
Mean (SD)	66.5 (18.34)	7.3 (14.71)	66.7 (13.46)	5.3 (15.60)		
Median	61.0	7.5	65.0	5.0		
Min, Max	40, 100	-20, 40	50, 90	-18, 30		
Week 4						
n	23	23	10	9		
Mean (SD)	67.1 (19.72)	7.9 (14.18)	66.7 (14.41)	1.0 (13.85)		
Median	70.0	10.0	67.5	-3.0		
Min, Max	30, 100	-20, 30	40, 90	-20, 25		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Table GERMANY.2.5.3.5

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Visual Analogue Scale (VAS) Score

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	19	19	7	6		
Mean (SD)	68.2 (21.04)	8.8 (19.19)	72.4 (11.75)	9.0 (15.23)		
Median	70.0	10.0	70.0	7.5		
Min, Max	25, 100	-25, 45	57, 90	-11, 30		
Week 12						
n	22	22	10	9		
Mean (SD)	74.6 (17.60)	15.0 (16.16)	70.0 (10.80)	5.8 (10.02)		
Median	75.0	10.0	70.0	2.0		
Min, Max	40, 100	-10, 48	50, 85	-10, 20		
Week 14						
n	22	22	10	9		
Mean (SD)	75.1 (17.67)	15.5 (15.48)	63.2 (26.11)	-2.9 (27.89)		
Median	77.5	17.5	74.5	10.0		
Min, Max	45, 100	-10, 51	7, 90	-73, 20		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.5

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Visual Analogue Scale (VAS) Score

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	22	22	9	8		
Mean (SD)	73.0 (21.58)	13.3 (18.98)	72.1 (15.46)	7.6 (8.28)		
Median	74.0	10.0	77.0	7.0		
Min, Max	15, 100	-35, 48	50, 90	0, 25		
Week 20						
n	17	17	9	9		
Mean (SD)	73.9 (16.41)	10.2 (15.02)	67.0 (19.35)	2.8 (13.91)		
Median	76.0	10.0	67.0	0.0		
Min, Max	50, 100	-10, 40	40, 91	-20, 25		
Week 24						
n	21	21	10	9		
Mean (SD)	73.3 (20.91)	12.3 (18.63)	62.2 (22.77)	-1.8 (15.85)		
Median	75.0	10.0	62.5	-3.0		
Min, Max	20, 100	-20, 50	20, 90	-20, 25		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.5
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Visual Analogue Scale (VAS) Score

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	8	8	27	27
Mean (SD)	73.4 (21.58)	12.3 (21.08)	55.4 (25.74)	-11.9 (30.66)	68.0 (23.89)	5.1 (26.21)
Median	75.0	10.0	62.0	-2.0	70.0	3.0
Min, Max	35, 100	-20, 50	7, 82	-68, 15	7, 100	-68, 50
Week 56						
n	14	14	7	6	21	20
Mean (SD)	63.3 (27.91)	2.9 (33.63)	65.6 (11.86)	-2.3 (8.52)	64.0 (23.45)	1.4 (28.27)
Median	65.0	9.0	65.0	0.0	65.0	0.0
Min, Max	0, 100	-98, 47	50, 80	-14, 10	0, 100	-98, 47
Week 72						
n	9	9	6	5	15	14
Mean (SD)	69.0 (21.71)	11.4 (15.95)	59.8 (27.15)	-14.8 (26.71)	65.3 (23.54)	2.1 (23.37)
Median	70.0	5.0	70.0	-2.0	70.0	1.0
Min, Max	45, 100	-3, 46	6, 80	-62, 0	6, 100	-62, 46

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Table GERMANY.2.5.3.5

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Visual Analogue Scale (VAS) Score

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	7	3	3	10	10
Mean (SD)	83.6 (13.14)	21.0 (14.09)	75.0 (5.00)	0.7 (11.02)	81.0 (11.74)	14.9 (16.00)
Median	80.0	20.0	75.0	0.0	80.0	13.5
Min, Max	60, 100	2, 45	70, 80	-10, 12	60, 100	-10, 45
Week 104						
n	3	3	1	1	4	4
Mean (SD)	78.3 (18.93)	10.7 (12.50)	65.0 (NA)	-10.0 (NA)	75.0 (16.83)	5.5 (14.53)
Median	70.0	5.0	65.0	-10.0	67.5	3.5
Min, Max	65, 100	2, 25	65, 65	-10, -10	65, 100	-10, 25

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.5

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Visual Analogue Scale (VAS) Score

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		15		48	
Mean (SD)	59.1 (20.28)		58.9 (22.85)		59.0 (20.87)	
Median	65.0		70.0		67.5	
Min, Max	7, 95		9, 90		7, 95	
Week 2						
n	33	33	16	15		
Mean (SD)	69.5 (17.12)	10.4 (21.87)	62.3 (16.32)	3.5 (24.04)		
Median	71.0	10.0	60.0	0.0		
Min, Max	7, 95	-67, 63	30, 90	-25, 81		
Week 4						
n	33	33	15	15		
Mean (SD)	70.8 (15.73)	11.8 (19.38)	62.1 (21.39)	3.1 (29.26)		
Median	75.0	10.0	70.0	0.0		
Min, Max	30, 91	-24, 55	30, 90	-50, 81		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.5

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Visual Analogue Scale (VAS) Score

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	25	25	12	12		
Mean (SD)	71.9 (19.73)	17.6 (17.57)	67.7 (20.42)	8.2 (29.98)		
Median	70.0	16.0	71.0	1.0		
Min, Max	8, 100	-5, 60	25, 90	-40, 81		
Week 12						
n	30	30	15	15		
Mean (SD)	72.0 (18.11)	13.7 (17.45)	63.4 (19.56)	4.5 (27.75)		
Median	70.0	10.0	70.0	0.0		
Min, Max	7, 97	-11, 65	30, 90	-35, 81		
Week 14						
n	31	31	13	13		
Mean (SD)	73.9 (12.28)	14.5 (18.61)	71.9 (13.16)	12.4 (24.01)		
Median	75.0	10.0	70.0	5.0		
Min, Max	45, 96	-10, 65	55, 90	-10, 81		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.5

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Visual Analogue Scale (VAS) Score

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	30	30	14	14		
Mean (SD)	70.5 (15.48)	11.5 (19.79)	72.7 (15.88)	12.4 (22.93)		
Median	70.0	8.5	71.5	7.5		
Min, Max	30, 95	-25, 65	40, 95	-10, 81		
Week 20						
n	30	30	11	11		
Mean (SD)	70.9 (15.05)	11.1 (20.90)	77.4 (7.61)	21.1 (25.42)		
Median	73.0	5.0	78.0	10.0		
Min, Max	30, 98	-20, 68	69, 90	-2, 81		
Week 24						
n	29	29	13	13		
Mean (SD)	74.1 (13.25)	15.3 (19.62)	77.7 (10.51)	18.2 (22.45)		
Median	75.0	9.0	80.0	12.0		
Min, Max	30, 96	-13, 68	60, 91	0, 82		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.5

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Visual Analogue Scale (VAS) Score

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	12	12	39	39
Mean (SD)	68.8 (21.73)	12.0 (28.56)	73.1 (16.57)	16.5 (22.58)	70.1 (20.16)	13.4 (26.65)
Median	75.0	10.0	70.0	10.0	73.0	10.0
Min, Max	7, 95	-67, 70	35, 98	-1, 81	7, 98	-67, 81
Week 56						
n	22	22	9	9	31	31
Mean (SD)	73.0 (15.29)	17.2 (21.78)	73.6 (19.04)	15.3 (28.67)	73.1 (16.14)	16.6 (23.49)
Median	78.0	10.5	80.0	5.0	80.0	10.0
Min, Max	40, 95	-11, 65	30, 90	-10, 81	30, 95	-11, 81
Week 72						
n	12	12	5	5	17	17
Mean (SD)	66.7 (27.41)	5.8 (34.69)	69.0 (21.91)	0.0 (23.72)	67.4 (25.25)	4.1 (31.23)
Median	75.0	5.0	80.0	-5.0	75.0	2.0
Min, Max	8, 95	-62, 70	30, 80	-20, 40	8, 95	-62, 70

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.3.5

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Visual Analogue Scale (VAS) Score

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	7	5	5	12	12
Mean (SD)	71.9 (12.08)	4.7 (15.51)	71.0 (10.25)	9.0 (17.46)	71.5 (10.86)	6.5 (15.72)
Median	75.0	5.0	75.0	10.0	75.0	7.5
Min, Max	50, 85	-20, 30	60, 80	-15, 30	50, 85	-20, 30
Week 104						
n	1	1	1	1	2	2
Mean (SD)	95.0 (NA)	15.0 (NA)	80.0 (NA)	30.0 (NA)	87.5 (10.61)	22.5 (10.61)
Median	95.0	15.0	80.0	30.0	87.5	22.5
Min, Max	95, 95	15, 15	80, 80	30, 30	80, 95	15, 30

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.6
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		15		50	
Mean (SD)	56.8 (17.77)		59.8 (21.57)		57.7 (18.81)	
Median	60.0		65.0		60.0	
Min, Max	7, 87		9, 90		7, 90	
Week 2						
n	35	35	15	15		
Mean (SD)	68.2 (15.58)	11.5 (16.42)	65.3 (12.74)	5.5 (24.75)		
Median	70.0	10.0	65.0	0.0		
Min, Max	40, 100	-20, 63	50, 90	-25, 81		
Week 4						
n	34	34	15	15		
Mean (SD)	68.7 (16.96)	12.0 (15.49)	66.5 (17.71)	6.7 (29.69)		
Median	70.5	10.0	65.0	0.0		
Min, Max	30, 95	-20, 45	40, 90	-50, 81		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.6
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	28	28	12	12		
Mean (SD)	64.7 (21.62)	9.4 (16.34)	72.3 (13.17)	11.3 (30.28)		
Median	70.0	10.5	75.0	5.0		
Min, Max	8, 100	-25, 40	50, 90	-40, 81		
Week 12						
n	32	32	15	15		
Mean (SD)	70.9 (19.56)	14.5 (14.34)	67.1 (16.49)	7.3 (26.51)		
Median	72.0	10.0	70.0	0.0		
Min, Max	7, 100	-10, 40	35, 90	-35, 81		
Week 14						
n	32	32	14	14		
Mean (SD)	71.7 (15.17)	15.2 (16.21)	70.3 (17.00)	9.1 (22.54)		
Median	75.0	11.0	74.5	7.5		
Min, Max	45, 100	-10, 58	40, 90	-10, 81		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Table GERMANY.2.5.3.6
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	31	31	14	14		
Mean (SD)	70.2 (18.82)	14.2 (17.49)	73.9 (15.11)	12.6 (20.93)		
Median	75.0	10.0	74.5	10.0		
Min, Max	15, 100	-35, 48	50, 90	0, 81		
Week 20						
n	29	29	13	13		
Mean (SD)	73.7 (13.34)	15.0 (18.25)	71.8 (16.95)	12.8 (23.11)		
Median	75.0	10.0	78.0	5.0		
Min, Max	50, 100	-10, 68	40, 90	-10, 81		
Week 24						
n	30	30	14	14		
Mean (SD)	72.3 (17.41)	14.6 (18.07)	70.0 (21.99)	8.8 (24.57)		
Median	75.0	11.0	76.0	1.0		
Min, Max	20, 100	-20, 58	20, 91	-20, 82		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.6
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	12	12	41	41
Mean (SD)	72.5 (17.08)	15.2 (18.84)	64.9 (26.42)	3.5 (36.49)	70.3 (20.21)	11.8 (25.37)
Median	75.0	10.0	67.0	10.0	73.0	10.0
Min, Max	35, 100	-20, 59	7, 98	-68, 81	7, 100	-68, 81
Week 56						
n	22	22	9	9	31	31
Mean (SD)	72.9 (18.86)	14.4 (19.84)	69.9 (15.33)	10.8 (29.60)	72.0 (17.71)	13.3 (22.63)
Median	77.5	11.5	65.0	0.0	76.0	10.0
Min, Max	40, 100	-11, 65	50, 90	-14, 81	40, 100	-14, 81
Week 72						
n	12	12	7	7	19	19
Mean (SD)	67.3 (20.94)	6.1 (21.25)	57.7 (28.79)	-14.1 (22.63)	63.8 (23.81)	-1.4 (23.39)
Median	71.0	5.0	73.0	-2.0	72.0	0.0
Min, Max	30, 96	-40, 46	6, 80	-62, 0	6, 96	-62, 46

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.6
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and
Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level
(< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	8	8	5	5	13	13
Mean (SD)	79.4 (13.48)	14.4 (19.54)	74.0 (8.22)	1.4 (10.71)	77.3 (11.66)	9.4 (17.44)
Median	80.0	12.5	75.0	0.0	80.0	10.0
Min, Max	50, 95	-20, 45	60, 80	-15, 12	50, 95	-20, 45
Week 104						
n	2	2	1	1	3	3
Mean (SD)	82.5 (17.68)	10.0 (7.07)	65.0 (NA)	-10.0 (NA)	76.7 (16.07)	3.3 (12.58)
Median	82.5	10.0	65.0	-10.0	70.0	5.0
Min, Max	70, 95	5, 15	65, 65	-10, -10	65, 95	-10, 15

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.6
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	62.9 (22.73)		62.8 (18.56)		62.9 (21.29)	
Median	70.0		70.0		70.0	
Min, Max	25, 98		30, 85		25, 98	
Week 2						
n	22	22	10	8		
Mean (SD)	68.2 (20.69)	5.3 (22.62)	61.6 (18.86)	1.4 (12.79)		
Median	73.0	9.0	60.0	0.0		
Min, Max	7, 100	-67, 55	30, 90	-20, 20		
Week 4						
n	22	22	10	9		
Mean (SD)	70.2 (18.44)	7.3 (20.03)	60.1 (20.47)	-4.9 (7.59)		
Median	75.0	3.0	70.0	-5.0		
Min, Max	30, 100	-24, 55	30, 80	-20, 5		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.6
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	16	16	7	6		
Mean (SD)	80.2 (12.62)	21.4 (20.34)	64.6 (23.62)	2.8 (11.67)		
Median	80.0	20.0	70.0	1.0		
Min, Max	60, 100	-10, 60	25, 90	-15, 20		
Week 12						
n	20	20	10	9		
Mean (SD)	76.7 (14.23)	14.0 (20.46)	64.5 (17.71)	1.1 (13.87)		
Median	73.0	6.0	70.0	0.0		
Min, Max	48, 100	-11, 65	30, 90	-20, 30		
Week 14						
n	21	21	9	8		
Mean (SD)	78.5 (13.01)	14.5 (19.08)	64.8 (24.29)	1.0 (32.61)		
Median	80.0	10.0	70.0	7.5		
Min, Max	50, 100	-10, 65	7, 90	-73, 40		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.6
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Visual Analogue Scale (VAS) Score

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	21	21	9	8		
Mean (SD)	73.4 (17.40)	9.4 (21.80)	70.3 (16.41)	7.3 (15.07)		
Median	70.0	1.0	72.0	3.5		
Min, Max	30, 100	-25, 65	40, 95	-10, 41		
Week 20						
n	18	18	7	7		
Mean (SD)	69.2 (18.42)	3.9 (18.11)	74.4 (10.21)	13.0 (23.31)		
Median	70.0	1.5	70.0	10.0		
Min, Max	30, 98	-20, 65	60, 91	-20, 51		
Week 24						
n	20	20	9	8		
Mean (SD)	75.9 (15.77)	13.2 (20.95)	72.4 (11.36)	12.1 (17.99)		
Median	75.0	5.0	71.0	16.0		
Min, Max	30, 100	-13, 68	60, 90	-20, 41		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.6
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	8	8	25	25
Mean (SD)	67.6 (27.91)	6.8 (34.02)	67.6 (14.39)	7.6 (13.78)	67.6 (24.08)	7.1 (28.76)
Median	80.0	5.0	70.0	2.5	70.0	5.0
Min, Max	7, 100	-67, 70	35, 82	-10, 30	7, 100	-67, 70
Week 56						
n	14	14	7	6	21	20
Mean (SD)	63.4 (24.16)	7.4 (36.91)	70.3 (18.77)	4.5 (13.47)	65.7 (22.28)	6.5 (31.33)
Median	70.0	9.5	75.0	1.0	70.0	3.5
Min, Max	0, 92	-98, 60	30, 87	-10, 30	0, 92	-98, 60
Week 72						
n	9	9	4	3	13	12
Mean (SD)	68.1 (30.08)	11.1 (35.95)	75.0 (5.77)	8.3 (27.54)	70.2 (24.95)	10.4 (32.85)
Median	75.0	5.0	75.0	-5.0	75.0	5.0
Min, Max	8, 100	-62, 70	70, 80	-10, 40	8, 100	-62, 70

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.6
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	3	3	9	9
Mean (SD)	75.5 (14.61)	10.8 (13.03)	70.0 (10.00)	13.3 (20.82)	73.7 (12.88)	11.7 (14.70)
Median	75.0	10.0	70.0	20.0	70.0	10.0
Min, Max	60, 100	-7, 30	60, 80	-10, 30	60, 100	-10, 30
Week 104						
n	2	2	1	1	3	3
Mean (SD)	82.5 (24.75)	13.5 (16.26)	80.0 (NA)	30.0 (NA)	81.7 (17.56)	19.0 (14.93)
Median	82.5	13.5	80.0	30.0	80.0	25.0
Min, Max	65, 100	2, 25	80, 80	30, 30	65, 100	2, 30

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.7
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Visual Analogue Scale (VAS) Score

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		10		31	
Mean (SD)	61.9 (19.26)		68.0 (17.03)		63.9 (18.51)	
Median	70.0		72.5		70.0	
Min, Max	25, 95		40, 90		25, 95	
Week 2						
n	21	21	11	9		
Mean (SD)	66.2 (21.73)	4.3 (23.21)	64.1 (10.91)	-1.7 (10.61)		
Median	70.0	0.0	60.0	0.0		
Min, Max	7, 100	-67, 40	50, 85	-25, 10		
Week 4						
n	20	20	11	10		
Mean (SD)	65.5 (17.21)	3.5 (17.15)	62.0 (16.12)	-7.8 (16.75)		
Median	66.0	0.0	70.0	-1.5		
Min, Max	30, 95	-24, 39	40, 80	-50, 10		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.7
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and
Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	14	14	6	5		
Mean (SD)	69.1 (18.12)	10.9 (19.22)	70.8 (14.97)	-2.0 (22.80)		
Median	67.5	1.0	70.0	0.0		
Min, Max	40, 100	-10, 40	50, 90	-40, 20		
Week 12						
n	17	17	11	10		
Mean (SD)	72.3 (18.21)	11.1 (17.42)	64.1 (16.40)	-4.5 (14.42)		
Median	70.0	10.0	70.0	0.0		
Min, Max	40, 100	-11, 40	35, 90	-35, 20		
Week 14						
n	18	18	10	9		
Mean (SD)	71.4 (15.68)	8.6 (16.48)	62.2 (25.79)	-7.6 (25.72)		
Median	72.0	2.0	65.0	0.0		
Min, Max	45, 100	-10, 40	7, 90	-73, 10		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.7
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Visual Analogue Scale (VAS) Score

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	17	17	11	10		
Mean (SD)	72.6 (16.63)	10.3 (21.19)	74.3 (14.49)	5.7 (5.81)		
Median	70.0	0.0	77.0	6.0		
Min, Max	45, 100	-25, 48	50, 95	0, 15		
Week 20						
n	15	15	8	8		
Mean (SD)	76.2 (13.61)	7.9 (20.86)	66.9 (16.24)	3.8 (14.82)		
Median	75.0	3.0	70.0	2.5		
Min, Max	50, 100	-10, 59	40, 90	-20, 25		
Week 24						
n	15	15	10	9		
Mean (SD)	71.5 (20.44)	7.1 (21.36)	68.7 (18.51)	1.9 (12.73)		
Median	75.0	1.0	67.5	0.0		
Min, Max	20, 100	-20, 50	30, 90	-20, 25		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.7
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Visual Analogue Scale (VAS) Score

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	9	9	26	26
Mean (SD)	68.2 (22.90)	10.2 (29.78)	63.9 (26.02)	-2.2 (26.09)	66.7 (23.59)	5.9 (28.66)
Median	75.0	10.0	70.0	8.0	70.0	9.0
Min, Max	7, 100	-67, 59	7, 98	-68, 20	7, 100	-68, 59
Week 56						
n	13	13	9	8	22	21
Mean (SD)	70.7 (18.75)	13.7 (23.71)	72.4 (13.04)	5.3 (11.85)	71.4 (16.32)	10.5 (20.10)
Median	70.0	10.0	70.0	1.0	70.0	9.0
Min, Max	40, 97	-20, 65	50, 90	-10, 30	40, 97	-20, 65
Week 72						
n	7	7	8	7	15	14
Mean (SD)	75.4 (19.89)	14.7 (21.91)	67.3 (16.36)	-7.4 (7.83)	71.1 (17.92)	3.6 (19.54)
Median	75.0	5.0	71.5	-5.0	75.0	0.0
Min, Max	35, 96	-10, 46	30, 80	-20, 0	30, 96	-20, 46

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.3.7

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Visual Analogue Scale (VAS) Score

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	5	5	5	5	10	10
Mean (SD)	77.6 (12.60)	15.6 (21.26)	72.0 (7.58)	-3.0 (9.75)	74.8 (10.24)	6.3 (18.42)
Median	75.0	5.0	75.0	0.0	75.0	2.5
Min, Max	63, 95	-7, 45	60, 80	-15, 10	60, 95	-15, 45
Week 104						
n	1	1	1	1	2	2
Mean (SD)	95.0 (NA)	15.0 (NA)	65.0 (NA)	-10.0 (NA)	80.0 (21.21)	2.5 (17.68)
Median	95.0	15.0	65.0	-10.0	80.0	2.5
Min, Max	95, 95	15, 15	65, 65	-10, -10	65, 95	-10, 15

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.7
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Visual Analogue Scale (VAS) Score

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		14		50	
Mean (SD)	57.5 (20.30)		55.9 (21.21)		57.1 (20.35)	
Median	60.0		66.5		60.0	
Min, Max	7, 98		9, 80		7, 98	
Week 2						
n	36	36	14	14		
Mean (SD)	69.4 (14.79)	11.9 (15.92)	63.6 (18.32)	7.8 (25.45)		
Median	70.0	10.0	65.0	3.0		
Min, Max	40, 100	-16, 63	30, 90	-20, 81		
Week 4						
n	36	36	14	14		
Mean (SD)	71.4 (17.39)	13.9 (16.63)	65.4 (21.00)	9.6 (26.69)		
Median	75.0	10.0	68.0	0.5		
Min, Max	30, 100	-20, 55	30, 90	-20, 81		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.
Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.7
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and
Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	30	30	13	13		
Mean (SD)	70.9 (21.30)	15.1 (18.47)	68.8 (19.10)	12.5 (26.17)		
Median	75.0	13.5	72.0	2.0		
Min, Max	8, 100	-25, 60	25, 90	-15, 81		
Week 12						
n	35	35	14	14		
Mean (SD)	73.5 (17.81)	15.8 (16.48)	67.6 (17.33)	11.7 (25.09)		
Median	73.0	11.0	70.0	1.0		
Min, Max	7, 100	-10, 65	30, 90	-20, 81		
Week 14						
n	35	35	13	13		
Mean (SD)	75.9 (14.01)	18.2 (16.92)	72.7 (13.02)	15.6 (22.79)		
Median	80.0	11.0	75.0	10.0		
Min, Max	45, 100	-5, 65	50, 90	-5, 81		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.7
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Visual Analogue Scale (VAS) Score

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	35	35	12	12		
Mean (SD)	71.0 (19.06)	13.2 (18.54)	70.8 (16.58)	14.8 (24.63)		
Median	75.0	10.0	72.0	7.5		
Min, Max	15, 100	-35, 65	40, 90	-10, 81		
Week 20						
n	32	32	12	12		
Mean (SD)	70.0 (16.06)	12.1 (17.97)	76.6 (12.81)	18.9 (25.28)		
Median	71.0	8.5	79.0	9.0		
Min, Max	30, 97	-20, 68	45, 91	-2, 81		
Week 24						
n	35	35	13	13		
Mean (SD)	74.7 (15.06)	17.0 (17.51)	72.7 (18.66)	15.6 (25.61)		
Median	75.0	11.0	74.0	12.0		
Min, Max	30, 100	-10, 68	20, 91	-20, 82		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.3.7

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Visual Analogue Scale (VAS) Score

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	11	11	40	40
Mean (SD)	72.2 (20.98)	13.2 (23.08)	67.7 (19.17)	11.2 (31.18)	71.0 (20.36)	12.7 (25.15)
Median	75.0	10.0	70.0	10.0	74.0	10.0
Min, Max	8, 100	-62, 70	30, 90	-50, 81	8, 100	-62, 81
Week 56						
n	23	23	7	7	30	30
Mean (SD)	68.3 (22.94)	10.5 (29.82)	67.0 (20.49)	11.7 (33.96)	68.0 (22.05)	10.8 (30.23)
Median	70.0	11.0	75.0	0.0	72.5	10.0
Min, Max	0, 100	-98, 60	30, 90	-14, 81	0, 100	-98, 81
Week 72						
n	14	14	3	3	17	17
Mean (SD)	63.8 (26.39)	5.0 (30.53)	55.3 (42.72)	-7.3 (51.39)	62.3 (28.38)	2.8 (33.33)
Median	70.0	5.0	80.0	0.0	70.0	5.0
Min, Max	8, 100	-62, 70	6, 80	-62, 40	6, 100	-62, 70

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.7

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Visual Analogue Scale (VAS) Score

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	9	9	3	3	12	12
Mean (SD)	77.8 (14.81)	11.3 (14.57)	73.3 (11.55)	20.7 (9.02)	76.7 (13.71)	13.7 (13.67)
Median	80.0	10.0	80.0	20.0	80.0	13.5
Min, Max	50, 100	-20, 30	60, 80	12, 30	50, 100	-20, 30
Week 104						
n	3	3	1	1	4	4
Mean (SD)	78.3 (18.93)	10.7 (12.50)	80.0 (NA)	30.0 (NA)	78.8 (15.48)	15.5 (14.06)
Median	70.0	5.0	80.0	30.0	75.0	15.0
Min, Max	65, 100	2, 25	80, 80	30, 30	65, 100	2, 30

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.3.8

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Visual Analogue Scale (VAS) Score

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	59.1 (18.78)		72.5 (5.00)		63.6 (16.57)	
Median	55.0		70.0		70.0	
Min, Max	35, 87		70, 80		35, 87	
Week 2						
n	8	8	4	4		
Mean (SD)	60.9 (18.02)	1.8 (12.62)	73.8 (12.50)	1.3 (13.15)		
Median	57.5	4.0	72.5	-2.5		
Min, Max	40, 90	-20, 20	60, 90	-10, 20		
Week 4						
n	8	8	4	4		
Mean (SD)	67.6 (22.60)	8.5 (20.09)	67.8 (9.67)	-4.8 (6.08)		
Median	72.0	7.5	65.5	-5.0		
Min, Max	30, 95	-20, 45	60, 80	-10, 1		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.
Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.3.8

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Visual Analogue Scale (VAS) Score

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	8	8	4	4		
Mean (SD)	64.4 (20.65)	5.3 (13.79)	75.0 (12.91)	2.5 (12.58)		
Median	56.0	8.0	75.0	0.0		
Min, Max	40, 95	-10, 25	60, 90	-10, 20		
Week 12						
n	8	8	4	4		
Mean (SD)	71.1 (16.92)	12.0 (15.69)	74.0 (4.90)	1.5 (5.97)		
Median	71.0	10.0	73.0	0.0		
Min, Max	50, 92	-10, 35	70, 80	-4, 10		
Week 14						
n	8	8	4	4		
Mean (SD)	70.5 (20.09)	11.4 (16.78)	75.3 (7.32)	2.8 (6.85)		
Median	75.0	9.5	77.5	2.5		
Min, Max	45, 95	-10, 45	65, 81	-5, 11		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Table GERMANY.2.5.3.8

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Visual Analogue Scale (VAS) Score

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	8	8	3	3		
Mean (SD)	63.5 (26.31)	4.4 (19.70)	75.0 (5.00)	1.7 (2.89)		
Median	65.0	9.0	75.0	0.0		
Min, Max	15, 95	-35, 25	70, 80	0, 5		
Week 20						
n	8	8	4	4		
Mean (SD)	69.8 (18.88)	10.6 (15.09)	79.0 (9.06)	6.5 (10.66)		
Median	71.0	8.0	78.0	3.5		
Min, Max	50, 97	-10, 36	69, 91	-2, 21		
Week 24						
n	8	8	4	4		
Mean (SD)	68.5 (21.77)	9.4 (16.09)	81.5 (6.61)	9.0 (8.87)		
Median	67.0	10.0	81.0	8.0		
Min, Max	40, 100	-20, 28	74, 90	0, 20		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Table GERMANY.2.5.3.8

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Visual Analogue Scale (VAS) Score

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	69.5 (26.71)	9.2 (23.57)	77.3 (6.43)	7.3 (6.43)	72.1 (21.72)	8.6 (18.93)
Median	76.0	11.5	80.0	10.0	80.0	10.0
Min, Max	35, 100	-20, 35	70, 82	0, 12	35, 100	-20, 35
Week 56						
n	4	4	1	1	5	5
Mean (SD)	73.8 (28.69)	4.5 (12.69)	60.0 (NA)	-10.0 (NA)	71.0 (25.59)	1.6 (12.76)
Median	77.5	4.0	60.0	-10.0	60.0	0.0
Min, Max	40, 100	-10, 20	60, 60	-10, -10	40, 100	-10, 20
Week 72						
n	1	1	0	0	1	1
Mean (SD)	47.0 (NA)	-3.0 (NA)			47.0 (NA)	-3.0 (NA)
Median	47.0	-3.0			47.0	-3.0
Min, Max	47, 47	-3, -3			47, 47	-3, -3

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.8

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Visual Analogue Scale (VAS) Score

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	49		20		69	
Mean (SD)	59.1 (20.22)		58.6 (21.28)		59.0 (20.37)	
Median	60.0		62.5		60.0	
Min, Max	7, 98		9, 90		7, 98	
Week 2						
n	49	49	21	19		
Mean (SD)	69.4 (17.36)	10.3 (19.79)	62.0 (15.20)	4.7 (22.67)		
Median	70.0	10.0	60.0	0.0		
Min, Max	7, 100	-67, 63	30, 90	-25, 81		
Week 4						
n	48	48	21	20		
Mean (SD)	69.6 (16.67)	10.4 (17.13)	63.2 (20.07)	3.8 (26.36)		
Median	71.0	10.0	70.0	0.0		
Min, Max	30, 100	-24, 55	30, 90	-50, 81		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.
Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.8

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	36	36	15	14		
Mean (SD)	71.6 (20.09)	15.7 (19.14)	67.9 (18.63)	10.1 (28.28)		
Median	72.0	13.5	70.0	6.0		
Min, Max	8, 100	-25, 60	25, 90	-40, 81		
Week 12						
n	44	44	21	20		
Mean (SD)	73.5 (18.09)	14.7 (17.09)	64.5 (17.74)	5.7 (24.52)		
Median	72.5	10.0	70.0	0.0		
Min, Max	7, 100	-11, 65	30, 90	-35, 81		
Week 14						
n	45	45	19	18		
Mean (SD)	75.1 (13.60)	15.6 (17.41)	66.6 (21.39)	6.9 (28.88)		
Median	75.0	11.0	70.0	10.0		
Min, Max	45, 100	-10, 65	7, 90	-73, 81		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.8

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Visual Analogue Scale (VAS) Score

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	44	44	20	19		
Mean (SD)	73.0 (16.25)	13.7 (19.09)	72.1 (16.42)	12.1 (19.91)		
Median	75.0	10.0	72.0	10.0		
Min, Max	30, 100	-25, 65	40, 95	-10, 81		
Week 20						
n	39	39	16	16		
Mean (SD)	72.5 (14.90)	10.8 (19.66)	71.1 (15.62)	14.4 (24.67)		
Median	75.0	5.0	72.5	7.5		
Min, Max	30, 100	-20, 68	40, 90	-20, 81		
Week 24						
n	42	42	19	18		
Mean (SD)	74.8 (15.69)	14.9 (19.64)	68.7 (19.26)	10.2 (24.17)		
Median	75.0	9.5	70.0	6.0		
Min, Max	20, 100	-15, 68	20, 91	-20, 82		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.3.8

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Visual Analogue Scale (VAS) Score

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	40	40	17	17	57	57
Mean (SD)	70.9 (21.07)	12.6 (26.00)	64.0 (23.22)	4.8 (31.58)	68.8 (21.75)	10.2 (27.72)
Median	75.0	10.0	69.0	8.0	70.0	10.0
Min, Max	7, 100	-67, 70	7, 98	-68, 81	7, 100	-68, 81
Week 56						
n	32	32	15	14	47	46
Mean (SD)	68.6 (20.69)	12.5 (28.79)	70.7 (16.66)	9.6 (24.34)	69.3 (19.34)	11.6 (27.28)
Median	70.0	10.5	75.0	1.0	70.0	10.0
Min, Max	0, 97	-98, 65	30, 90	-14, 81	0, 97	-98, 81
Week 72						
n	20	20	11	10	31	30
Mean (SD)	68.7 (24.72)	8.8 (28.38)	64.0 (24.15)	-7.4 (25.06)	67.0 (24.22)	3.4 (27.98)
Median	73.5	5.0	73.0	-3.5	73.0	2.0
Min, Max	8, 100	-62, 70	6, 80	-62, 40	6, 100	-62, 70

EQ-5D-3L is Three-level EuroQoL 5 dimensions.
Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.8

Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Visual Analogue Scale (VAS) Score

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	77.7 (13.56)	12.9 (16.56)	72.5 (8.45)	5.9 (15.08)	75.8 (12.01)	10.3 (16.04)
Median	80.0	10.0	75.0	5.0	80.0	10.0
Min, Max	50, 100	-20, 45	60, 80	-15, 30	50, 100	-20, 45
Week 104						
n	4	4	2	2	6	6
Mean (SD)	82.5 (17.56)	11.8 (10.44)	72.5 (10.61)	10.0 (28.28)	79.2 (15.30)	11.2 (15.04)
Median	82.5	10.0	72.5	10.0	75.0	10.0
Min, Max	65, 100	2, 25	65, 80	-10, 30	65, 100	-10, 30

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Table GERMANY.2.5.3.9
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and
Long Term Extension by Race
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	61.7 (19.42)		57.9 (24.17)		60.5 (20.69)	
Median	67.5		70.0		70.0	
Min, Max	25, 96		9, 80		9, 96	
Week 2						
n	22	22	10	10		
Mean (SD)	67.2 (15.90)	5.5 (16.07)	72.0 (13.78)	14.1 (26.51)		
Median	70.0	2.0	72.5	5.0		
Min, Max	40, 91	-20, 55	50, 90	-10, 81		
Week 4						
n	22	22	10	10		
Mean (SD)	72.2 (16.54)	10.5 (18.69)	72.1 (17.66)	14.2 (29.81)		
Median	76.0	10.0	75.5	0.5		
Min, Max	30, 95	-20, 55	35, 90	-10, 81		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.9
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Visual Analogue Scale (VAS) Score

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	18	18	10	10		
Mean (SD)	70.6 (17.99)	10.4 (18.39)	73.5 (19.73)	15.6 (28.78)		
Median	70.0	7.5	80.0	5.0		
Min, Max	40, 97	-10, 60	25, 90	-15, 81		
Week 12						
n	20	20	10	10		
Mean (SD)	74.9 (14.90)	12.5 (18.44)	71.6 (15.31)	13.7 (26.87)		
Median	75.0	10.0	78.0	0.0		
Min, Max	50, 97	-10, 65	40, 90	-4, 81		
Week 14						
n	20	20	9	9		
Mean (SD)	75.1 (16.29)	12.7 (17.36)	75.1 (15.25)	15.2 (25.43)		
Median	80.0	9.5	80.0	10.0		
Min, Max	45, 97	-10, 65	50, 90	-5, 81		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.
Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Table GERMANY.2.5.3.9
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and
Long Term Extension by Race
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	20	20	8	8		
Mean (SD)	72.7 (20.44)	10.3 (19.17)	74.4 (16.78)	15.8 (26.73)		
Median	75.0	10.0	77.5	10.0		
Min, Max	15, 97	-35, 65	50, 90	0, 81		
Week 20						
n	20	20	9	9		
Mean (SD)	74.1 (17.02)	11.7 (16.22)	76.8 (14.50)	16.9 (26.29)		
Median	76.0	9.0	78.0	8.0		
Min, Max	45, 97	-10, 65	45, 91	-2, 81		
Week 24						
n	20	20	9	9		
Mean (SD)	74.4 (16.95)	12.0 (18.53)	73.0 (22.42)	13.1 (29.02)		
Median	76.5	9.0	80.0	10.0		
Min, Max	40, 100	-20, 68	20, 91	-20, 82		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.9
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and
Long Term Extension by Race
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	7	7	27	27
Mean (SD)	76.2 (17.95)	13.8 (20.47)	72.4 (20.93)	12.6 (38.57)	75.2 (18.42)	13.5 (25.49)
Median	80.0	9.5	80.0	10.0	80.0	10.0
Min, Max	35, 100	-20, 70	30, 90	-50, 81	30, 100	-50, 81
Week 56						
n	16	16	5	5	21	21
Mean (SD)	76.3 (17.03)	14.7 (19.09)	70.0 (25.50)	14.2 (38.25)	74.8 (18.84)	14.6 (23.79)
Median	80.0	12.0	80.0	0.0	80.0	10.0
Min, Max	40, 100	-11, 60	30, 90	-10, 81	30, 100	-11, 81
Week 72						
n	9	9	3	3	12	12
Mean (SD)	71.9 (21.43)	13.0 (31.48)	80.0 (0.00)	13.3 (23.09)	73.9 (18.64)	13.1 (28.59)
Median	72.0	5.0	80.0	0.0	80.0	3.5
Min, Max	30, 95	-40, 70	80, 80	0, 40	30, 95	-40, 70

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.9
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and
Long Term Extension by Race
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	7	2	2	9	9
Mean (SD)	75.7 (13.05)	10.7 (17.18)	70.0 (14.14)	10.0 (14.14)	74.4 (12.61)	10.6 (15.70)
Median	80.0	10.0	70.0	10.0	80.0	10.0
Min, Max	50, 90	-20, 30	60, 80	0, 20	50, 90	-20, 30
Week 104						
n	2	2	0	0	2	2
Mean (SD)	82.5 (17.68)	10.0 (7.07)			82.5 (17.68)	10.0 (7.07)
Median	82.5	10.0			82.5	10.0
Min, Max	70, 95	5, 15			70, 95	5, 15

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.9
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Visual Analogue Scale (VAS) Score

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		10		38	
Mean (SD)	59.7 (19.38)		67.3 (17.66)		61.7 (19.01)	
Median	60.0		69.0		65.0	
Min, Max	25, 98		30, 90		25, 98	
Week 2						
n	28	28	10	9		
Mean (SD)	71.1 (16.59)	11.4 (14.01)	61.6 (13.13)	-4.1 (12.41)		
Median	70.5	10.0	60.0	0.0		
Min, Max	40, 100	-15, 40	36, 85	-25, 10		
Week 4						
n	27	27	10	10		
Mean (SD)	68.6 (17.19)	8.9 (15.10)	59.7 (18.01)	-7.6 (16.97)		
Median	70.0	10.0	67.5	-3.0		
Min, Max	30, 100	-20, 39	30, 80	-50, 10		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.
Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.9
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and
Long Term Extension by Race
Modified Randomized Set

Visual Analogue Scale (VAS) Score

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	21	21	7	7		
Mean (SD)	70.9 (18.62)	14.8 (20.03)	63.4 (16.31)	-3.4 (18.11)		
Median	75.0	15.0	60.0	0.0		
Min, Max	25, 100	-25, 45	40, 85	-40, 15		
Week 12						
n	25	25	10	10		
Mean (SD)	74.1 (16.83)	15.2 (16.82)	66.5 (15.64)	-0.8 (17.94)		
Median	70.0	10.0	67.5	0.0		
Min, Max	40, 100	-11, 48	35, 90	-35, 30		
Week 14						
n	26	26	10	10		
Mean (SD)	75.2 (13.90)	15.1 (16.82)	65.1 (24.00)	-2.2 (29.25)		
Median	75.5	15.5	70.0	0.0		
Min, Max	45, 100	-10, 51	7, 90	-73, 40		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.9
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Visual Analogue Scale (VAS) Score

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	25	25	10	10		
Mean (SD)	72.0 (16.42)	12.3 (19.71)	77.2 (11.50)	9.9 (13.64)		
Median	70.0	7.0	74.5	3.0		
Min, Max	40, 100	-25, 48	60, 95	0, 41		
Week 20						
n	21	21	8	8		
Mean (SD)	73.2 (12.33)	9.9 (17.38)	72.9 (13.03)	10.6 (22.60)		
Median	70.0	5.0	77.5	7.5		
Min, Max	45, 100	-10, 59	50, 90	-20, 51		
Week 24						
n	23	23	10	10		
Mean (SD)	75.3 (16.12)	14.5 (18.39)	75.5 (11.38)	8.2 (17.69)		
Median	75.0	9.0	76.0	1.0		
Min, Max	20, 100	-15, 50	60, 90	-20, 41		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.3.9
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and
Long Term Extension by Race
Modified Randomized Set

Visual Analogue Scale (VAS) Score

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	9	9	28	28
Mean (SD)	70.9 (21.20)	12.7 (27.30)	61.4 (26.35)	-3.9 (25.78)	67.9 (22.93)	7.4 (27.50)
Median	73.0	10.0	69.0	0.0	70.0	6.5
Min, Max	8, 100	-62, 59	7, 98	-68, 20	7, 100	-68, 59
Week 56						
n	15	15	7	7	22	22
Mean (SD)	63.9 (23.64)	5.3 (35.61)	71.6 (12.09)	1.9 (14.15)	66.3 (20.69)	4.2 (30.08)
Median	70.0	10.0	75.0	0.0	70.0	3.5
Min, Max	0, 97	-98, 65	54, 87	-14, 30	0, 97	-98, 65
Week 72						
n	9	9	6	6	15	15
Mean (SD)	62.7 (29.44)	0.7 (28.20)	60.7 (27.59)	-15.7 (23.35)	61.9 (27.72)	-5.9 (26.79)
Median	70.0	5.0	71.5	-7.5	70.0	0.0
Min, Max	8, 100	-62, 46	6, 80	-62, 0	6, 100	-62, 46

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.9
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and
Long Term Extension by Race
Modified Randomized Set

Visual Analogue Scale (VAS) Score

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	4	4	10	10
Mean (SD)	83.0 (13.11)	14.2 (18.43)	75.0 (4.08)	-3.3 (11.93)	79.8 (10.87)	7.2 (17.81)
Median	80.0	10.0	75.0	-5.0	80.0	6.0
Min, Max	63, 100	-7, 45	70, 80	-15, 12	63, 100	-15, 45
Week 104						
n	1	1	1	1	2	2
Mean (SD)	100.0 (NA)	2.0 (NA)	65.0 (NA)	-10.0 (NA)	82.5 (24.75)	-4.0 (8.49)
Median	100.0	2.0	65.0	-10.0	82.5	-4.0
Min, Max	100, 100	2, 2	65, 65	-10, -10	65, 100	-10, 2

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Table GERMANY.2.5.3.9
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Visual Analogue Scale (VAS) Score

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		4		11	
Mean (SD)	48.7 (22.78)		52.5 (12.58)		50.1 (19.04)	
Median	50.0		50.0		50.0	
Min, Max	7, 74		40, 70		7, 74	
Week 2						
n	7	7	5	4		
Mean (SD)	59.7 (24.93)	11.0 (39.23)	52.0 (14.83)	-2.5 (12.58)		
Median	70.0	11.0	50.0	0.0		
Min, Max	7, 80	-67, 63	30, 70	-20, 10		
Week 4						
n	7	7	5	4		
Mean (SD)	62.9 (21.38)	14.1 (23.18)	56.0 (19.49)	-2.5 (12.58)		
Median	60.0	20.0	50.0	0.0		
Min, Max	30, 90	-24, 43	30, 80	-20, 10		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.3.9
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Visual Analogue Scale (VAS) Score

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	5	5	2	1		
Mean (SD)	66.8 (35.17)	21.4 (12.12)	70.0 (0.00)	20.0 (NA)		
Median	70.0	26.0	70.0	20.0		
Min, Max	8, 100	1, 30	70, 70	20, 20		
Week 12						
n	7	7	5	4		
Mean (SD)	64.6 (27.35)	15.9 (12.79)	54.0 (18.17)	-2.5 (17.08)		
Median	70.0	20.0	60.0	-5.0		
Min, Max	7, 90	-4, 30	30, 70	-20, 20		
Week 14						
n	7	7	4	3		
Mean (SD)	69.4 (13.06)	20.7 (19.57)	60.0 (16.33)	6.7 (5.77)		
Median	70.0	20.0	60.0	10.0		
Min, Max	50, 90	-4, 58	40, 80	0, 10		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.
Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.9
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and
Long Term Extension by Race
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 16						
n	7	7	5	4		
Mean (SD)	66.4 (19.09)	17.7 (19.89)	60.0 (15.81)	2.5 (9.57)		
Median	70.0	20.0	60.0	5.0		
Min, Max	30, 90	-10, 48	40, 80	-10, 10		
Week 20						
n	6	6	3	3		
Mean (SD)	60.8 (17.68)	10.7 (32.28)	60.0 (17.32)	6.7 (11.55)		
Median	67.5	0.5	70.0	0.0		
Min, Max	30, 75	-20, 68	40, 70	0, 20		
Week 24						
n	7	7	4	3		
Mean (SD)	67.0 (18.80)	18.3 (24.66)	55.0 (17.32)	6.7 (15.28)		
Median	70.0	19.0	60.0	10.0		
Min, Max	30, 89	-13, 58	30, 70	-10, 20		

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.9
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and Long Term Extension by Race Modified Randomized Set

Visual Analogue Scale (VAS) Score

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	7	7	4	4	11	11
Mean (SD)	54.4 (26.54)	5.7 (35.28)	65.0 (12.91)	12.5 (12.58)	58.3 (22.39)	8.2 (28.39)
Median	54.0	10.0	65.0	10.0	60.0	10.0
Min, Max	7, 90	-67, 47	50, 80	0, 30	7, 90	-67, 47
Week 56						
n	5	5	4	3	9	8
Mean (SD)	62.4 (23.43)	21.0 (22.29)	67.5 (12.58)	13.3 (15.28)	64.7 (18.47)	18.1 (19.14)
Median	60.0	22.0	70.0	10.0	70.0	16.0
Min, Max	40, 92	0, 53	50, 80	0, 30	40, 92	0, 53
Week 72						
n	3	3	2	1	5	4
Mean (SD)	70.0 (22.91)	16.7 (10.41)	50.0 (28.28)	-20.0 (NA)	62.0 (24.14)	7.5 (20.21)
Median	75.0	20.0	50.0	-20.0	70.0	12.5
Min, Max	45, 90	5, 25	30, 70	-20, -20	30, 90	-20, 25

EQ-5D-3L is Three-level EuroQoL 5 dimensions.
Visual Analogue Scale (VAS) Score is reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.3.9
Summary of Actual Values and Change from Baseline in EQ-5D-3L Scores During Treatment Period 1 and Period 2 and
Long Term Extension by Race
Modified Randomized Set

Visual Analogue Scale (VAS) Score

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	1	1	2	2	3	3
Mean (SD)	60.0 (NA)	20.0 (NA)	70.0 (14.14)	20.0 (14.14)	66.7 (11.55)	20.0 (10.00)
Median	60.0	20.0	70.0	20.0	60.0	20.0
Min, Max	60, 60	20, 20	60, 80	10, 30	60, 80	10, 30
Week 104						
n	1	1	1	1	2	2
Mean (SD)	65.0 (NA)	25.0 (NA)	80.0 (NA)	30.0 (NA)	72.5 (10.61)	27.5 (3.54)
Median	65.0	25.0	80.0	30.0	72.5	27.5
Min, Max	65, 65	25, 25	80, 80	30, 30	65, 80	25, 30

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

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Table GERMANY.2.5.1.2
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.97 (3.855)	10.86 (6.154)	-4.89 (7.134)	0.4980
	95% CI for LS MEAN	(-1.91, 13.84)	(-1.69, 23.41)	(-19.47, 9.68)	
Week 4	LS Mean (SEM)	7.62 (3.242)	8.49 (4.907)	-0.87 (5.737)	0.8812
	95% CI for LS MEAN	(0.97, 14.27)	(-1.59, 18.56)	(-12.64, 10.91)	
Week 8	LS Mean (SEM)	9.57 (3.485)	14.14 (5.397)	-4.58 (6.287)	0.4738
	95% CI for LS MEAN	(2.39, 16.75)	(3.02, 25.27)	(-17.55, 8.40)	
Week 12	LS Mean (SEM)	10.32 (3.274)	8.49 (4.937)	1.83 (5.785)	0.7542
	95% CI for LS MEAN	(3.61, 17.03)	(-1.64, 18.62)	(-10.04, 13.70)	

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.2
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	10.70 (2.321)	0.65 (3.538)	10.04 (4.133)	0.0193
	95% CI for LS MEAN	(6.02, 15.37)	(-6.47, 7.78)	(1.71, 18.37)	
Week 4	LS Mean (SEM)	12.23 (2.902)	0.05 (4.367)	12.17 (5.164)	0.0231
	95% CI for LS MEAN	(6.38, 18.08)	(-8.75, 8.86)	(1.75, 22.59)	
Week 8	LS Mean (SEM)	11.54 (3.426)	2.61 (5.176)	8.92 (6.141)	0.1533
	95% CI for LS MEAN	(4.63, 18.44)	(-7.81, 13.03)	(-3.45, 21.30)	
Week 12	LS Mean (SEM)	15.16 (2.742)	4.25 (4.021)	10.90 (4.779)	0.0278
	95% CI for LS MEAN	(9.63, 20.68)	(-3.86, 12.37)	(1.25, 20.55)	

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.1.2
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.0734
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EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.1.3
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by Age at Informed Consent
(< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	11.41 (2.641)	4.43 (3.967)	6.98 (4.629)	0.1374
	95% CI for LS MEAN	(6.12, 16.70)	(-3.52, 12.38)	(-2.30, 16.26)	
Week 4	LS Mean (SEM)	11.49 (2.889)	4.21 (4.321)	7.28 (5.073)	0.1572
	95% CI for LS MEAN	(5.70, 17.27)	(-4.45, 12.87)	(-2.89, 17.45)	
Week 8	LS Mean (SEM)	13.46 (3.108)	5.01 (4.711)	8.45 (5.529)	0.1327
	95% CI for LS MEAN	(7.22, 19.69)	(-4.44, 14.46)	(-2.65, 19.55)	
Week 12	LS Mean (SEM)	14.80 (2.584)	6.88 (3.845)	7.92 (4.490)	0.0833
	95% CI for LS MEAN	(9.62, 19.98)	(-0.83, 14.58)	(-1.08, 16.93)	

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.3
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by Age at Informed Consent
(< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	2.83 (3.236)	2.90 (5.605)	-0.06 (6.480)	0.9922
	95% CI for LS MEAN	(-3.98, 9.65)	(-8.82, 14.61)	(-13.64, 13.51)	
Week 4	LS Mean (SEM)	8.21 (2.819)	-1.14 (4.662)	9.35 (5.487)	0.1059
	95% CI for LS MEAN	(2.28, 14.14)	(-10.94, 8.67)	(-2.19, 20.89)	
Week 8	LS Mean (SEM)	5.19 (3.998)	8.12 (6.522)	-2.93 (7.660)	0.7062
	95% CI for LS MEAN	(-3.16, 13.53)	(-5.52, 21.75)	(-18.94, 13.08)	
Week 12	LS Mean (SEM)	9.36 (4.242)	1.36 (6.725)	7.99 (7.944)	0.3281
	95% CI for LS MEAN	(0.45, 18.26)	(-12.84, 15.56)	(-8.74, 24.73)	

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.3
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by Age at Informed Consent
(< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.6172
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EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.4
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	9.40 (3.425)	-3.62 (5.107)	13.02 (6.125)	0.0409
	95% CI for LS MEAN	(2.43, 16.36)	(-14.00, 6.75)	(0.57, 25.47)	
Week 4	LS Mean (SEM)	7.20 (3.206)	-5.54 (4.754)	12.74 (5.710)	0.0325
	95% CI for LS MEAN	(0.68, 13.73)	(-15.21, 4.13)	(1.13, 24.36)	
Week 8	LS Mean (SEM)	7.83 (4.085)	-0.09 (6.325)	7.92 (7.500)	0.3005
	95% CI for LS MEAN	(-0.60, 16.25)	(-13.04, 12.86)	(-7.47, 23.30)	
Week 12	LS Mean (SEM)	14.38 (2.937)	-2.37 (4.343)	16.76 (5.220)	0.0031
	95% CI for LS MEAN	(8.40, 20.37)	(-11.24, 6.49)	(6.11, 27.41)	

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.4
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	9.10 (2.623)	10.88 (4.217)	-1.78 (4.817)	0.7136
	95% CI for LS MEAN	(3.80, 14.39)	(2.37, 19.38)	(-11.51, 7.95)	
Week 4	LS Mean (SEM)	13.38 (2.905)	10.19 (4.563)	3.19 (5.294)	0.5504
	95% CI for LS MEAN	(7.51, 19.24)	(0.97, 19.41)	(-7.51, 13.89)	
Week 8	LS Mean (SEM)	13.34 (3.389)	12.23 (5.255)	1.12 (6.155)	0.8569
	95% CI for LS MEAN	(6.51, 20.18)	(1.60, 22.85)	(-11.33, 13.56)	
Week 12	LS Mean (SEM)	13.11 (2.964)	12.27 (4.577)	0.84 (5.340)	0.8761
	95% CI for LS MEAN	(7.13, 19.09)	(3.02, 21.52)	(-9.95, 11.63)	

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.4
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.0495
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EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.5
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.76 (3.016)	4.03 (4.966)	1.73 (5.411)	0.7511
	95% CI for LS MEAN	(-0.38, 11.91)	(-6.06, 14.12)	(-9.30, 12.77)	
Week 4	LS Mean (SEM)	7.51 (3.149)	1.25 (4.931)	6.26 (5.496)	0.2648
	95% CI for LS MEAN	(1.07, 13.95)	(-8.86, 11.35)	(-5.03, 17.55)	
Week 8	LS Mean (SEM)	6.94 (3.719)	7.37 (6.178)	-0.43 (6.932)	0.9511
	95% CI for LS MEAN	(-0.64, 14.52)	(-5.21, 19.94)	(-14.59, 13.73)	
Week 12	LS Mean (SEM)	12.68 (2.941)	6.03 (4.571)	6.66 (5.057)	0.1990
	95% CI for LS MEAN	(6.67, 18.70)	(-3.34, 15.39)	(-3.72, 17.03)	

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.5
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	10.93 (2.852)	3.85 (4.230)	7.09 (5.098)	0.1715
	95% CI for LS MEAN	(5.19, 16.68)	(-4.68, 12.37)	(-3.19, 17.36)	
Week 4	LS Mean (SEM)	12.27 (2.936)	3.51 (4.354)	8.75 (5.248)	0.1025
	95% CI for LS MEAN	(6.35, 18.18)	(-5.26, 12.29)	(-1.83, 19.33)	
Week 8	LS Mean (SEM)	14.25 (3.339)	5.14 (4.876)	9.11 (5.907)	0.1306
	95% CI for LS MEAN	(7.51, 20.99)	(-4.70, 14.99)	(-2.82, 21.03)	
Week 12	LS Mean (SEM)	13.42 (3.028)	4.85 (4.396)	8.58 (5.335)	0.1152
	95% CI for LS MEAN	(7.32, 19.53)	(-4.02, 13.72)	(-2.18, 19.33)	

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.5
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors
of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.5080
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EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.6
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors
of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	11.02 (2.368)	6.98 (3.625)	4.04 (4.336)	0.3569
	95% CI for LS MEAN	(6.25, 15.79)	(-0.32, 14.28)	(-4.70, 12.77)	
Week 4	LS Mean (SEM)	12.47 (2.857)	8.12 (4.349)	4.35 (5.209)	0.4078
	95% CI for LS MEAN	(6.72, 18.22)	(-0.65, 16.88)	(-6.14, 14.85)	
Week 8	LS Mean (SEM)	7.56 (3.015)	11.49 (4.610)	-3.93 (5.513)	0.4792
	95% CI for LS MEAN	(1.49, 13.62)	(2.22, 20.76)	(-15.02, 7.15)	
Week 12	LS Mean (SEM)	12.82 (2.875)	8.72 (4.335)	4.10 (5.207)	0.4353
	95% CI for LS MEAN	(7.03, 18.61)	(-0.03, 17.46)	(-6.40, 14.60)	

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.6
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors
of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.10 (4.048)	1.15 (6.472)	4.96 (7.315)	0.5033
	95% CI for LS MEAN	(-2.16, 14.36)	(-12.03, 14.32)	(-10.00, 19.91)	
Week 4	LS Mean (SEM)	8.06 (3.511)	-4.11 (5.323)	12.17 (6.014)	0.0530
	95% CI for LS MEAN	(0.88, 15.24)	(-15.01, 6.79)	(-0.17, 24.50)	
Week 8	LS Mean (SEM)	18.09 (3.696)	-0.70 (5.771)	18.79 (6.513)	0.0087
	95% CI for LS MEAN	(10.46, 25.73)	(-12.62, 11.21)	(5.28, 32.31)	
Week 12	LS Mean (SEM)	14.31 (3.279)	1.89 (4.861)	12.42 (5.486)	0.0319
	95% CI for LS MEAN	(7.59, 21.03)	(-8.08, 11.86)	(1.16, 23.68)	

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.6
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors
of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.4760
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EQ-5D-3L is Three-level EuroQoL 5 dimensions.
Visual Analogue Scale (VAS) Score is reported.
LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.
The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.
Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).
P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.
Baseline is defined as the last non-missing value prior to the first dose of study drug.
Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.1.7
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by Background C5 Inhibitor Therapy
(Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	4.23 (3.994)	0.50 (6.156)	3.72 (7.165)	0.6072
	95% CI for LS MEAN	(-3.96, 12.41)	(-12.05, 13.05)	(-10.93, 18.38)	
Week 4	LS Mean (SEM)	5.06 (3.583)	-5.31 (5.310)	10.37 (6.231)	0.1081
	95% CI for LS MEAN	(-2.30, 12.42)	(-16.23, 5.60)	(-2.44, 23.19)	
Week 8	LS Mean (SEM)	5.65 (4.120)	6.16 (6.606)	-0.50 (7.637)	0.9479
	95% CI for LS MEAN	(-2.84, 14.15)	(-7.33, 19.65)	(-16.16, 15.15)	
Week 12	LS Mean (SEM)	10.72 (3.384)	-2.01 (4.863)	12.73 (5.740)	0.0352
	95% CI for LS MEAN	(3.79, 17.65)	(-12.00, 7.98)	(0.95, 24.52)	

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.7
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by Background C5 Inhibitor Therapy
(Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	11.50 (2.418)	6.31 (3.799)	5.20 (4.426)	0.2466
	95% CI for LS MEAN	(6.64, 16.37)	(-1.34, 13.96)	(-3.72, 14.11)	
Week 4	LS Mean (SEM)	13.48 (2.746)	8.09 (4.334)	5.38 (5.062)	0.2933
	95% CI for LS MEAN	(7.95, 19.00)	(-0.63, 16.81)	(-4.81, 15.57)	
Week 8	LS Mean (SEM)	13.91 (3.333)	8.20 (5.199)	5.70 (6.124)	0.3571
	95% CI for LS MEAN	(7.19, 20.63)	(-2.30, 18.70)	(-6.67, 18.08)	
Week 12	LS Mean (SEM)	14.68 (2.821)	10.24 (4.435)	4.44 (5.189)	0.3967
	95% CI for LS MEAN	(8.99, 20.36)	(1.29, 19.18)	(-6.03, 14.91)	

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.1.7
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by Background C5 Inhibitor Therapy
(Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.5736
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EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.8
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	13.06 (20.177)	0.25 (11.013)	12.81 (27.956)	0.6612
	95% CI for LS MEAN	(-34.61, 60.72)	(-30.90, 31.39)	(-53.93, 79.56)	
Week 4	LS Mean (SEM)	19.81 (20.336)	-5.75 (11.584)	25.56 (28.300)	0.3940
	95% CI for LS MEAN	(-27.38, 67.00)	(-35.15, 23.64)	(-40.26, 91.38)	
Week 8	LS Mean (SEM)	16.56 (20.359)	1.50 (11.665)	15.06 (28.350)	0.6116
	95% CI for LS MEAN	(-31.03, 64.15)	(-31.29, 34.28)	(-51.95, 82.08)	
Week 12	LS Mean (SEM)	23.31 (20.119)	0.50 (10.799)	22.81 (27.831)	0.4384
	95% CI for LS MEAN	(-23.94, 70.55)	(-25.40, 26.40)	(-42.49, 88.12)	

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese Patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.8

Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	10.66 (2.317)	3.67 (3.629)	6.99 (4.203)	0.1011
	95% CI for LS MEAN	(6.03, 15.29)	(-3.57, 10.91)	(-1.40, 15.38)	
Week 4	LS Mean (SEM)	11.34 (2.408)	3.49 (3.688)	7.86 (4.312)	0.0732
	95% CI for LS MEAN	(6.54, 16.15)	(-3.88, 10.85)	(-0.76, 16.47)	
Week 8	LS Mean (SEM)	13.01 (2.791)	6.01 (4.349)	7.00 (5.088)	0.1738
	95% CI for LS MEAN	(7.44, 18.59)	(-2.68, 14.70)	(-3.17, 17.17)	
Week 12	LS Mean (SEM)	13.89 (2.454)	5.39 (3.699)	8.51 (4.348)	0.0550
	95% CI for LS MEAN	(8.99, 18.79)	(-2.01, 12.78)	(-0.19, 17.20)	

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese Patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.8

Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.2767
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EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese Patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.9
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.36 (3.489)	11.60 (4.851)	-5.24 (5.536)	0.3521
	95% CI for LS MEAN	(-0.78, 13.50)	(1.66, 21.54)	(-16.60, 6.12)	
Week 4	LS Mean (SEM)	11.31 (3.882)	11.70 (5.469)	-0.39 (6.318)	0.9516
	95% CI for LS MEAN	(3.39, 19.23)	(0.52, 22.88)	(-13.33, 12.55)	
Week 8	LS Mean (SEM)	11.03 (4.073)	13.10 (5.616)	-2.07 (6.556)	0.7550
	95% CI for LS MEAN	(2.73, 19.33)	(1.62, 24.58)	(-15.49, 11.35)	
Week 12	LS Mean (SEM)	13.31 (3.578)	11.20 (4.905)	2.11 (5.643)	0.7109
	95% CI for LS MEAN	(6.01, 20.62)	(1.16, 21.24)	(-9.45, 13.67)	

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.9
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	10.86 (2.165)	-2.79 (3.860)	13.65 (4.412)	0.0039
	95% CI for LS MEAN	(6.45, 15.27)	(-10.63, 5.05)	(4.69, 22.61)	
Week 4	LS Mean (SEM)	9.22 (2.728)	-5.59 (4.597)	14.81 (5.345)	0.0091
	95% CI for LS MEAN	(3.67, 14.77)	(-14.94, 3.77)	(3.93, 25.68)	
Week 8	LS Mean (SEM)	10.55 (3.513)	-3.45 (6.035)	13.99 (6.979)	0.0537
	95% CI for LS MEAN	(3.37, 17.72)	(-15.75, 8.85)	(-0.23, 28.22)	
Week 12	LS Mean (SEM)	13.07 (2.890)	1.21 (4.793)	11.86 (5.597)	0.0425
	95% CI for LS MEAN	(7.17, 18.96)	(-8.58, 11.00)	(0.43, 23.28)	

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.9
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	24.73 (17.486)	2.75 (23.040)	21.98 (28.867)	0.4670
	95% CI for LS MEAN	(-15.13, 64.60)	(-49.81, 55.32)	(-43.89, 87.84)	
Week 4	LS Mean (SEM)	27.87 (11.262)	2.75 (14.755)	25.12 (18.472)	0.2116
	95% CI for LS MEAN	(1.88, 53.87)	(-31.37, 36.87)	(-17.61, 67.85)	
Week 8	LS Mean (SEM)	33.28 (6.825)	29.66 (10.723)	3.62 (12.633)	0.7822
	95% CI for LS MEAN	(17.02, 49.55)	(4.90, 54.42)	(-26.02, 33.27)	
Week 12	LS Mean (SEM)	29.59 (7.234)	2.75 (9.344)	26.83 (11.675)	0.0533
	95% CI for LS MEAN	(12.75, 46.43)	(-19.10, 24.61)	(-0.50, 54.17)	

EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.1.9
Change from Baseline in EQ-5D-3L Scores During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.1046
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EQ-5D-3L is Three-level EuroQoL 5 dimensions.

Visual Analogue Scale (VAS) Score is reported.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EQ-5D-3L score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.8 Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms (WPAI:ANS)

1.8.1 Rücklaufquote des WPAI:ANS

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Table GERMANY.2.5.5.10
WPAI:ANS Compliance Rate During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit	Statistics	D->D (N=57)	P->D (N=26)
Baseline	Expected Patients Per Visit	57	26
	Patients Per Visit	57	26
	Patients Who Completed Question, n (%)	55 (96.5)	24 (92.3)
Week 2	Expected Patients Per Visit	57	26
	Patients Per Visit	57	26
	Patients Who Completed Question, n (%)	51 (89.5)	23 (88.5)
Week 4	Expected Patients Per Visit	57	25
	Patients Per Visit	57	25
	Patients Who Completed Question, n (%)	57 (100)	24 (96.0)
Week 8	Expected Patients Per Visit	57	25
	Patients Per Visit	57	25
	Patients Who Completed Question, n (%)	45 (78.9)	18 (72.0)
Week 12	Expected Patients Per Visit	55	25
	Patients Per Visit	54	25
	Patients Who Completed Question, n (%)	51 (92.7)	24 (96.0)
Week 24	Expected Patients Per Visit	55	24
	Patients Per Visit	55	24
	Patients Who Completed Question, n (%)	46 (83.6)	22 (91.7)

Percentages are based on the total number of expected patients in each group.

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0.

All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported. For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.5.10
WPAI:ANS Compliance Rate During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Visit	Statistics	D->D (N=57)	P->D (N=26)
Week 40	Expected Patients Per Visit	46	22
	Patients Per Visit	46	22
	Patients Who Completed Question, n (%)	41 (89.1)	18 (81.8)
Week 56	Expected Patients Per Visit	36	18
	Patients Per Visit	36	18
	Patients Who Completed Question, n (%)	31 (86.1)	14 (77.8)
Week 72	Expected Patients Per Visit	21	12
	Patients Per Visit	21	12
	Patients Who Completed Question, n (%)	18 (85.7)	11 (91.7)
Week 88	Expected Patients Per Visit	14	8
	Patients Per Visit	14	8
	Patients Who Completed Question, n (%)	13 (92.9)	6 (75.0)
Week 104	Expected Patients Per Visit	4	2
	Patients Per Visit	4	2
	Patients Who Completed Question, n (%)	3 (75.0)	2 (100)

Percentages are based on the total number of expected patients in each group.

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0.

All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported. For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:50:50

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.8 Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms (WPAI:ANS)

1.8.2 Veränderung des WPAI:ANS gegenüber Baseline

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Table GERMANY.2.5.5.1
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	55		24		79	
Mean (SD)	4.327 (2.5170)		4.917 (2.5693)		4.506 (2.5312)	
Median	4.0		5.5		5.0	
Min, Max	0, 9		0, 9		0, 9	
Week 2						
n	51	51	23	22		
Mean (SD)	3.588 (2.4509)	-0.745 (2.3820)	4.696 (2.6873)	-0.318 (1.5240)		
Median	3.0	0.0	4.0	0.0		
Min, Max	0, 8	-9, 4	0, 8	-3, 3		
Week 4						
n	57	55	24	24		
Mean (SD)	3.088 (2.5793)	-1.236 (2.6734)	4.292 (2.4223)	-0.625 (1.7647)		
Median	3.0	-1.0	4.0	-1.0		
Min, Max	0, 8	-9, 5	0, 8	-5, 3		

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0.
All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported. For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which indicate Anemic symptoms completely prevented patients from doing daily activities.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Change from baseline is summarized only for patients who have data at baseline and the specified time point. Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:54:16

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Table GERMANY.2.5.5.1
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	45	43	18	18		
Mean (SD)	2.889 (2.6044)	-1.326 (2.8927)	4.389 (2.9334)	-0.111 (2.1933)		
Median	3.0	-1.0	4.0	0.0		
Min, Max	0, 8	-7, 7	0, 8	-5, 4		
Week 12						
n	51	49	24	24		
Mean (SD)	2.569 (2.5788)	-1.694 (2.8152)	4.417 (2.7806)	-0.500 (2.9192)		
Median	2.0	-2.0	4.0	-1.0		
Min, Max	0, 8	-9, 5	0, 9	-5, 9		
Week 24						
n	46	46	22	22		
Mean (SD)	2.283 (2.2771)	-1.913 (2.3835)	3.364 (2.5175)	-1.364 (2.3206)		
Median	2.0	-2.0	2.5	-1.0		
Min, Max	0, 8	-9, 4	0, 9	-7, 2		

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0.
All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported. For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which indicate Anemic symptoms completely prevented patients from doing daily activities.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Change from baseline is summarized only for patients who have data at baseline and the specified time point. Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.5.1

Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	41	41	18	18	59	59
Mean (SD)	2.585 (2.5591)	-1.366 (2.5569)	2.167 (2.2295)	-2.722 (2.3715)	2.458 (2.4518)	-1.780 (2.5601)
Median	2.0	-2.0	1.5	-2.5	2.0	-2.0
Min, Max	0, 9	-7, 5	0, 7	-8, 0	0, 9	-8, 5
Week 56						
n	31	31	14	13	45	44
Mean (SD)	2.516 (2.5413)	-1.871 (2.3908)	3.000 (2.6602)	-2.154 (3.1845)	2.667 (2.5584)	-1.955 (2.6144)
Median	2.0	-2.0	1.5	-2.0	2.0	-2.0
Min, Max	0, 8	-8, 3	0, 8	-7, 5	0, 8	-8, 5
Week 72						
n	18	18	11	11	29	29
Mean (SD)	3.056 (2.6228)	-1.278 (2.9065)	3.636 (3.0421)	-2.091 (2.4680)	3.276 (2.7503)	-1.586 (2.7323)
Median	3.0	-1.0	3.0	-2.0	3.0	-1.0
Min, Max	0, 7	-7, 3	0, 8	-6, 3	0, 8	-7, 3

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0.

All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported. For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which indicate Anemic symptoms completely prevented patients from doing daily activities.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point. Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:54:16

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Table GERMANY.2.5.5.1

Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	13	13	6	6	19	19
Mean (SD)	1.538 (1.7614)	-3.000 (2.9439)	2.500 (1.3784)	-3.333 (1.5055)	1.842 (1.6754)	-3.105 (2.5363)
Median	1.0	-3.0	2.5	-3.0	1.0	-3.0
Min, Max	0, 5	-9, 1	1, 4	-6, -2	0, 5	-9, 1
Week 104						
n	3	3	2	2	5	5
Mean (SD)	1.667 (2.8868)	-3.000 (5.2915)	3.000 (0.0000)	-4.000 (1.4142)	2.200 (2.1679)	-3.400 (3.8471)
Median	0.0	-1.0	3.0	-4.0	3.0	-3.0
Min, Max	0, 5	-9, 1	3, 3	-5, -3	0, 5	-9, 1

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0.

All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported. For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activities.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point. Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

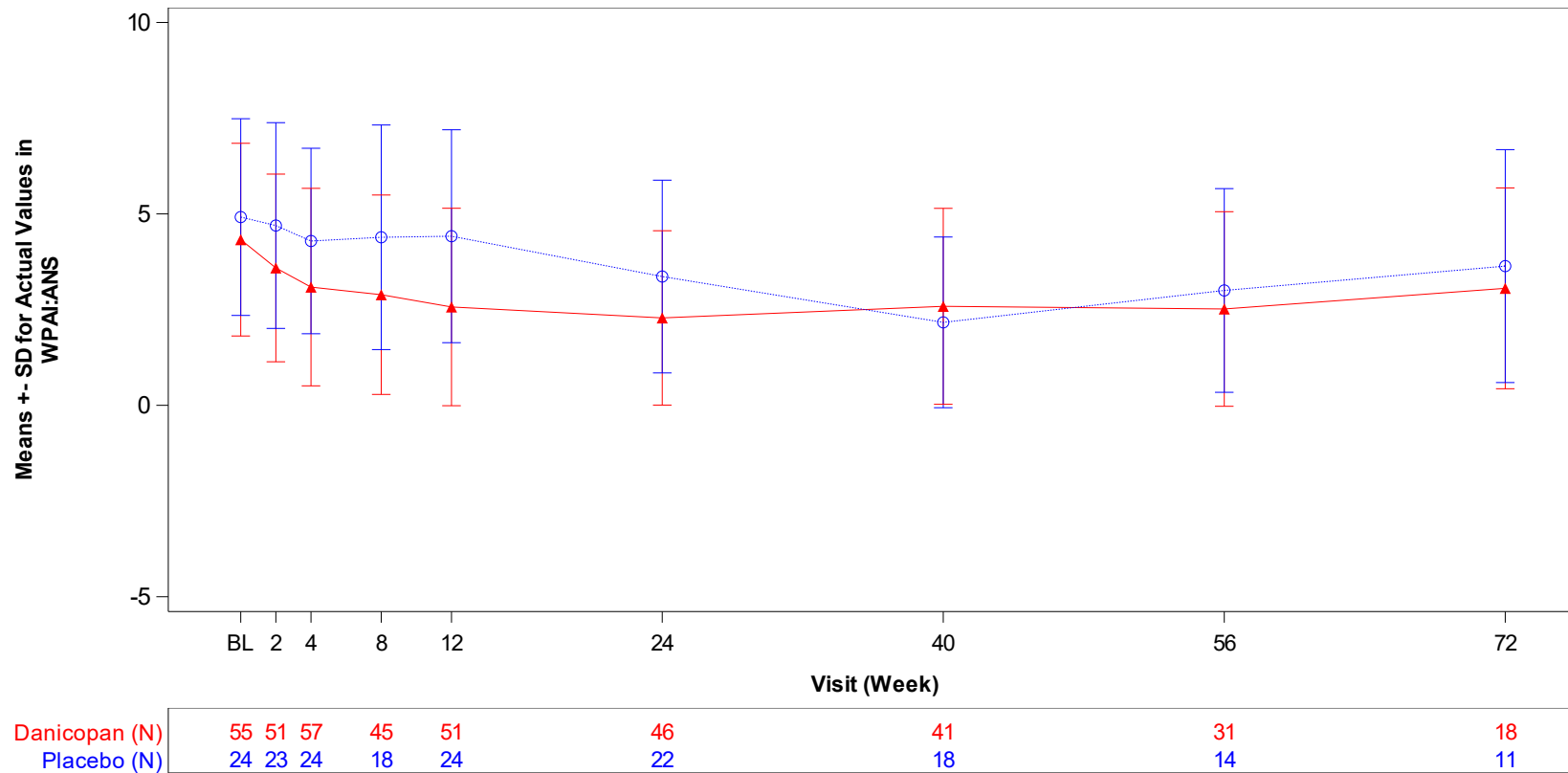
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Figure GERMANY.2.2.8.3
Plots of Mean +- SD for Actual Values in WPAI:ANS During 72 Weeks
Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.
WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.
For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

Source: ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.9.1
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Visit	Statistics	Danicopan (N = 55)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.94 (0.275)	-0.22 (0.419)	-0.72 (0.494)	0.1515
	95% CI for LS MEAN	(-1.49, -0.39)	(-1.06, 0.61)	(-1.70, 0.27)	
Week 4	LS Mean (SEM)	-1.38 (0.295)	-0.53 (0.446)	-0.85 (0.529)	0.1115
	95% CI for LS MEAN	(-1.97, -0.79)	(-1.42, 0.36)	(-1.91, 0.20)	
Week 8	LS Mean (SEM)	-1.60 (0.356)	-0.11 (0.545)	-1.49 (0.646)	0.0241
	95% CI for LS MEAN	(-2.31, -0.89)	(-1.20, 0.98)	(-2.78, -0.20)	
Week 12	LS Mean (SEM)	-1.79 (0.343)	-0.40 (0.500)	-1.39 (0.602)	0.0238
	95% CI for LS MEAN	(-2.48, -1.11)	(-1.40, 0.59)	(-2.59, -0.19)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). An Unstructured covariance matrix was used.

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:54:13

Data cutoff date of 31Mar2023

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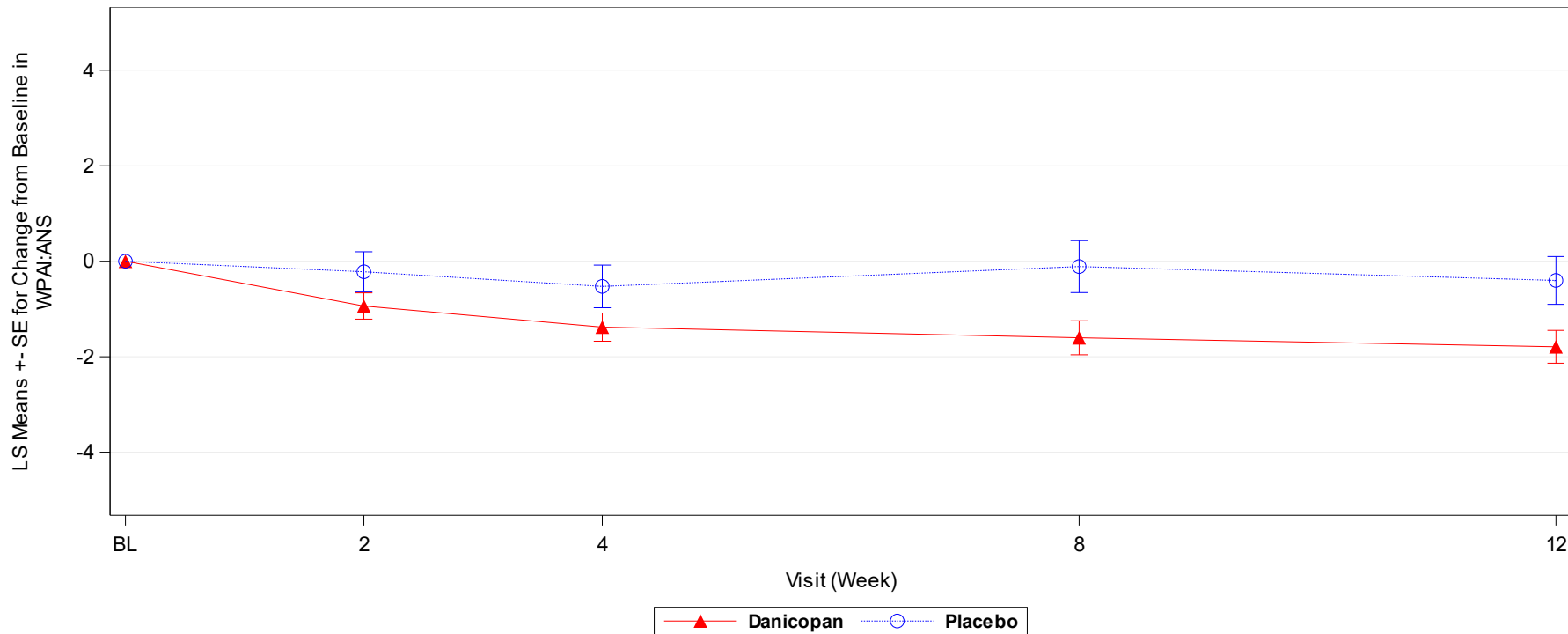
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Figure GERMANY.2.2.8.1
Plots of LS Means \pm SE for Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:31:46

Data cutoff date of 31Mar2023

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

1 Morbidität

1.8 Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms (WPAI:ANS)

1.8.2 Veränderung des WPAI:ANS gegenüber Baseline

1.8.2.1 Subgruppenanalysen – Veränderung des WPAI:ANS gegenüber Baseline

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Table GERMANY.2.5.5.2
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Sex
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean (SD)	4.1 (2.61)		3.1 (2.03)		3.8 (2.47)	
Median	4.0		2.0		4.0	
Min, Max	0, 9		0, 6		0, 9	
Week 2						
n	23	23	8	8		
Mean (SD)	3.6 (2.35)	-0.5 (1.90)	3.0 (2.00)	0.3 (1.83)		
Median	3.0	0.0	3.0	0.0		
Min, Max	0, 8	-5, 2	0, 7	-3, 3		
Week 4						
n	23	23	9	9		
Mean (SD)	3.3 (2.60)	-0.8 (2.71)	3.1 (2.62)	0.0 (1.32)		
Median	4.0	-1.0	3.0	0.0		
Min, Max	0, 8	-5, 5	0, 8	-2, 2		

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0.
All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported. For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Change from baseline is summarized only for patients who have data at baseline and the specified time point. Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:54:16

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Table GERMANY.2.5.5.2
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Sex
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	19	19	7	7		
Mean (SD)	2.8 (2.81)	-0.9 (3.25)	2.4 (2.76)	0.1 (1.95)		
Median	2.0	-1.0	2.0	0.0		
Min, Max	0, 8	-7, 7	0, 8	-2, 4		
Week 12						
n	21	21	9	9		
Mean (SD)	2.3 (2.71)	-1.6 (2.94)	3.9 (2.80)	0.8 (3.49)		
Median	1.0	-2.0	3.0	1.0		
Min, Max	0, 8	-7, 3	0, 9	-3, 9		
Week 24						
n	20	20	9	9		
Mean (SD)	1.9 (1.98)	-2.1 (2.25)	1.9 (0.93)	-1.2 (1.48)		
Median	2.0	-2.5	2.0	-1.0		
Min, Max	0, 8	-6, 2	0, 3	-4, 0		

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0.
All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported. For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.
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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Change from baseline is summarized only for patients who have data at baseline and the specified time point. Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:54:16

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Table GERMANY.2.5.5.2
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Sex
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	8	8	29	29
Mean (SD)	2.7 (2.52)	-1.5 (2.73)	1.3 (0.71)	-2.3 (1.98)	2.3 (2.25)	-1.7 (2.54)
Median	2.0	-2.0	1.0	-1.5	2.0	-2.0
Min, Max	0, 7	-7, 4	0, 2	-6, 0	0, 7	-7, 4
Week 56						
n	16	16	5	5	21	21
Mean (SD)	2.7 (2.94)	-1.8 (2.41)	2.2 (2.77)	-1.8 (4.21)	2.6 (2.84)	-1.8 (2.81)
Median	1.5	-2.0	1.0	-3.0	1.0	-2.0
Min, Max	0, 8	-7, 3	0, 7	-6, 5	0, 8	-7, 5
Week 72						
n	7	7	2	2	9	9
Mean (SD)	4.1 (2.85)	-0.9 (3.44)	0.0 (0.00)	-5.0 (1.41)	3.2 (3.07)	-1.8 (3.53)
Median	5.0	0.0	0.0	-5.0	3.0	-2.0
Min, Max	0, 7	-7, 3	0, 0	-6, -4	0, 7	-7, 3

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0.
All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported. For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:54:16

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ALXN2040-PNH-301

Table GERMANY.2.5.5.2
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Sex
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	5	5	2	2	7	7
Mean (SD)	2.2 (1.79)	-3.0 (2.55)	2.5 (2.12)	-2.5 (0.71)	2.3 (1.70)	-2.9 (2.12)
Median	2.0	-3.0	2.5	-2.5	2.0	-3.0
Min, Max	0, 4	-6, 0	1, 4	-3, -2	0, 4	-6, 0

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Table GERMANY.2.5.5.2
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Sex
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		15		47	
Mean (SD)	4.5 (2.48)		6.0 (2.27)		5.0 (2.49)	
Median	4.5		6.0		5.0	
Min, Max	0, 9		1, 9		0, 9	
Week 2						
n	28	28	15	14		
Mean (SD)	3.6 (2.57)	-1.0 (2.73)	5.6 (2.61)	-0.6 (1.28)		
Median	3.5	0.0	7.0	-0.5		
Min, Max	0, 8	-9, 4	1, 8	-3, 2		
Week 4						
n	34	32	15	15		
Mean (SD)	3.0 (2.60)	-1.5 (2.65)	5.0 (2.07)	-1.0 (1.93)		
Median	2.5	-1.5	5.0	-1.0		
Min, Max	0, 8	-9, 3	2, 8	-5, 3		

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Table GERMANY.2.5.5.2
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Sex
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	26	24	11	11		
Mean (SD)	2.9 (2.50)	-1.7 (2.60)	5.6 (2.38)	-0.3 (2.41)		
Median	3.0	-1.5	7.0	0.0		
Min, Max	0, 8	-6, 4	1, 8	-5, 4		
Week 12						
n	30	28	15	15		
Mean (SD)	2.7 (2.52)	-1.8 (2.77)	4.7 (2.81)	-1.3 (2.31)		
Median	2.0	-1.5	4.0	-1.0		
Min, Max	0, 8	-9, 5	0, 9	-5, 2		
Week 24						
n	26	26	13	13		
Mean (SD)	2.6 (2.47)	-1.8 (2.52)	4.4 (2.79)	-1.5 (2.82)		
Median	2.0	-2.0	5.0	-1.0		
Min, Max	0, 8	-9, 4	0, 9	-7, 2		

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Table GERMANY.2.5.5.2
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Sex
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	10	10	30	30
Mean (SD)	2.5 (2.67)	-1.3 (2.43)	2.9 (2.77)	-3.1 (2.69)	2.6 (2.66)	-1.9 (2.62)
Median	2.0	-1.5	2.5	-3.0	2.0	-2.0
Min, Max	0, 9	-6, 5	0, 7	-8, 0	0, 9	-8, 5
Week 56						
n	15	15	9	8	24	23
Mean (SD)	2.3 (2.13)	-2.0 (2.45)	3.4 (2.65)	-2.4 (2.67)	2.8 (2.35)	-2.1 (2.47)
Median	2.0	-2.0	3.0	-1.5	2.0	-2.0
Min, Max	0, 6	-8, 1	1, 8	-7, 0	0, 8	-8, 1
Week 72						
n	11	11	9	9	20	20
Mean (SD)	2.4 (2.34)	-1.5 (2.66)	4.4 (2.74)	-1.4 (2.19)	3.3 (2.68)	-1.5 (2.40)
Median	2.0	-1.0	3.0	-1.0	3.0	-1.0
Min, Max	0, 6	-5, 3	1, 8	-4, 3	0, 8	-5, 3

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Table GERMANY.2.5.5.2
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Sex
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	8	8	4	4	12	12
Mean (SD)	1.1 (1.73)	-3.0 (3.34)	2.5 (1.29)	-3.8 (1.71)	1.6 (1.68)	-3.3 (2.83)
Median	0.5	-2.5	2.5	-3.5	1.0	-3.5
Min, Max	0, 5	-9, 1	1, 4	-6, -2	0, 5	-9, 1
Week 104						
n	3	3	2	2	5	5
Mean (SD)	1.7 (2.89)	-3.0 (5.29)	3.0 (0.00)	-4.0 (1.41)	2.2 (2.17)	-3.4 (3.85)
Median	0.0	-1.0	3.0	-4.0	3.0	-3.0
Min, Max	0, 5	-9, 1	3, 3	-5, -3	0, 5	-9, 1

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Table GERMANY.2.5.5.3
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	40		18		58	
Mean (SD)	4.5 (2.58)		5.7 (2.22)		4.9 (2.52)	
Median	5.0		6.0		5.0	
Min, Max	0, 9		2, 9		0, 9	
Week 2						
n	38	38	19	18		
Mean (SD)	3.5 (2.59)	-0.9 (2.48)	5.3 (2.45)	-0.5 (1.58)		
Median	3.0	0.0	6.0	-0.5		
Min, Max	0, 8	-9, 4	2, 8	-3, 3		
Week 4						
n	41	40	18	18		
Mean (SD)	3.2 (2.71)	-1.4 (2.82)	4.6 (2.50)	-1.1 (1.64)		
Median	3.0	-1.5	4.0	-1.0		
Min, Max	0, 8	-9, 5	0, 8	-5, 2		

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Table GERMANY.2.5.5.3
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	32	31	14	14		
Mean (SD)	2.5 (2.30)	-1.8 (2.65)	5.3 (2.64)	-0.1 (2.48)		
Median	2.5	-2.0	6.0	-0.5		
Min, Max	0, 7	-7, 2	0, 8	-5, 4		
Week 12						
n	38	37	18	18		
Mean (SD)	2.6 (2.72)	-1.8 (2.93)	4.6 (2.59)	-1.1 (2.24)		
Median	2.0	-2.0	4.0	-1.0		
Min, Max	0, 8	-9, 5	0, 9	-5, 2		
Week 24						
n	35	35	16	16		
Mean (SD)	2.3 (2.25)	-2.1 (2.51)	3.8 (2.62)	-1.8 (2.52)		
Median	2.0	-2.0	3.0	-1.0		
Min, Max	0, 8	-9, 4	0, 9	-7, 2		

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Table GERMANY.2.5.5.3
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	14	14	43	43
Mean (SD)	2.4 (2.80)	-1.5 (2.86)	2.5 (2.41)	-3.0 (2.25)	2.5 (2.65)	-2.0 (2.74)
Median	1.0	-2.0	2.0	-3.0	2.0	-2.0
Min, Max	0, 9	-7, 5	0, 7	-8, 0	0, 9	-8, 5
Week 56						
n	21	21	12	11	33	32
Mean (SD)	2.3 (2.73)	-2.4 (2.64)	3.4 (2.64)	-2.0 (3.19)	2.7 (2.71)	-2.3 (2.79)
Median	1.0	-2.0	2.5	-2.0	1.0	-2.0
Min, Max	0, 8	-8, 3	1, 8	-7, 5	0, 8	-8, 5
Week 72						
n	12	12	9	9	21	21
Mean (SD)	3.5 (2.75)	-1.3 (3.28)	3.6 (2.74)	-2.2 (1.56)	3.5 (2.68)	-1.7 (2.67)
Median	3.5	-1.5	3.0	-2.0	3.0	-2.0
Min, Max	0, 7	-7, 3	0, 7	-4, 0	0, 7	-7, 3

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Table GERMANY.2.5.5.3

Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	7	5	5	12	12
Mean (SD)	0.9 (1.46)	-4.6 (2.82)	2.2 (1.30)	-3.6 (1.52)	1.4 (1.51)	-4.2 (2.33)
Median	0.0	-5.0	2.0	-3.0	1.0	-4.5
Min, Max	0, 4	-9, -1	1, 4	-6, -2	0, 4	-9, -1
Week 104						
n	2	2	2	2	4	4
Mean (SD)	0.0 (0.00)	-5.0 (5.66)	3.0 (0.00)	-4.0 (1.41)	1.5 (1.73)	-4.5 (3.42)
Median	0.0	-5.0	3.0	-4.0	1.5	-4.0
Min, Max	0, 0	-9, -1	3, 3	-5, -3	0, 3	-9, -1

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Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.5.3

Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	15		6		21	
Mean (SD)	3.9 (2.36)		2.7 (2.34)		3.5 (2.36)	
Median	4.0		2.0		4.0	
Min, Max	0, 8		0, 6		0, 8	
Week 2						
n	13	13	4	4		
Mean (SD)	3.9 (2.06)	-0.2 (2.03)	1.8 (1.71)	0.5 (1.00)		
Median	4.0	1.0	1.5	0.0		
Min, Max	0, 8	-5, 2	0, 4	0, 2		
Week 4						
n	16	15	6	6		
Mean (SD)	2.8 (2.26)	-0.9 (2.29)	3.5 (2.17)	0.8 (1.33)		
Median	3.5	-1.0	4.0	0.0		
Min, Max	0, 7	-5, 3	0, 6	0, 3		

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0.

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Table GERMANY.2.5.5.3

Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	13	12	4	4		
Mean (SD)	3.8 (3.17)	-0.1 (3.23)	1.3 (1.26)	0.0 (0.82)		
Median	4.0	-0.5	1.0	0.0		
Min, Max	0, 8	-5, 7	0, 3	-1, 1		
Week 12						
n	13	12	6	6		
Mean (SD)	2.5 (2.22)	-1.3 (2.49)	3.8 (3.49)	1.2 (4.22)		
Median	2.0	-1.0	3.0	0.0		
Min, Max	0, 7	-6, 3	0, 9	-3, 9		
Week 24						
n	11	11	6	6		
Mean (SD)	2.2 (2.48)	-1.4 (1.91)	2.3 (2.07)	-0.3 (1.37)		
Median	1.0	-1.0	2.0	0.0		
Min, Max	0, 8	-4, 2	0, 6	-3, 1		

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Table GERMANY.2.5.5.3
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	12	12	4	4	16	16
Mean (SD)	2.9 (1.93)	-1.0 (1.65)	1.0 (0.82)	-1.8 (2.87)	2.4 (1.90)	-1.2 (1.94)
Median	3.0	-1.0	1.0	-0.5	2.0	-0.5
Min, Max	0, 5	-3, 1	0, 2	-6, 0	0, 5	-6, 1
Week 56						
n	10	10	2	2	12	12
Mean (SD)	2.9 (2.18)	-0.8 (1.32)	0.5 (0.71)	-3.0 (4.24)	2.5 (2.20)	-1.2 (1.95)
Median	3.0	-0.5	0.5	-3.0	2.0	-0.5
Min, Max	0, 6	-3, 1	0, 1	-6, 0	0, 6	-6, 1
Week 72						
n	6	6	2	2	8	8
Mean (SD)	2.2 (2.32)	-1.3 (2.25)	4.0 (5.66)	-1.5 (6.36)	2.6 (3.02)	-1.4 (3.07)
Median	1.5	-0.5	4.0	-1.5	1.5	-0.5
Min, Max	0, 5	-5, 1	0, 8	-6, 3	0, 8	-6, 3

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Table GERMANY.2.5.5.3

Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	1	1	7	7
Mean (SD)	2.3 (1.86)	-1.2 (1.94)	4.0 (NA)	-2.0 (NA)	2.6 (1.81)	-1.3 (1.80)
Median	2.0	-0.5	4.0	-2.0	2.0	-1.0
Min, Max	0, 5	-4, 1	4, 4	-2, -2	0, 5	-4, 1
Week 104						
n	1	1	0	0	1	1
Mean (SD)	5.0 (NA)	1.0 (NA)			5.0 (NA)	1.0 (NA)
Median	5.0	1.0			5.0	1.0
Min, Max	5, 5	1, 1			5, 5	1, 1

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Table GERMANY.2.5.5.4
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Region
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		12		35	
Mean (SD)	4.7 (2.64)		5.5 (1.83)		4.9 (2.40)	
Median	5.0		6.0		5.0	
Min, Max	0, 9		2, 8		0, 9	
Week 2						
n	20	20	11	11		
Mean (SD)	4.0 (2.58)	-0.8 (2.24)	4.7 (2.45)	-0.8 (1.66)		
Median	3.0	0.0	3.0	-1.0		
Min, Max	0, 8	-5, 2	2, 8	-3, 2		
Week 4						
n	25	23	12	12		
Mean (SD)	3.2 (2.71)	-1.4 (2.74)	4.8 (2.21)	-0.7 (1.56)		
Median	4.0	-1.0	4.5	-1.0		
Min, Max	0, 8	-6, 4	2, 8	-3, 2		

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Table GERMANY.2.5.5.4
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Region
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	19	17	7	7		
Mean (SD)	2.9 (2.88)	-1.8 (3.51)	5.4 (2.30)	0.4 (2.23)		
Median	3.0	-2.0	5.0	0.0		
Min, Max	0, 8	-7, 7	2, 8	-3, 4		
Week 12						
n	22	20	12	12		
Mean (SD)	2.7 (2.93)	-2.0 (3.14)	4.7 (2.35)	-0.8 (2.37)		
Median	2.0	-2.0	4.0	-0.5		
Min, Max	0, 8	-7, 5	0, 8	-5, 2		
Week 24						
n	17	17	11	11		
Mean (SD)	2.4 (2.65)	-2.2 (2.83)	3.9 (2.51)	-1.5 (2.77)		
Median	2.0	-3.0	3.0	-1.0		
Min, Max	0, 8	-6, 4	0, 8	-7, 2		

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by Region
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	14	14	9	9	23	23
Mean (SD)	3.6 (2.85)	-1.2 (3.09)	2.9 (2.15)	-3.0 (1.87)	3.3 (2.57)	-1.9 (2.78)
Median	4.0	-2.0	2.0	-3.0	3.0	-2.0
Min, Max	0, 9	-7, 5	0, 7	-7, -1	0, 9	-7, 5
Week 56						
n	9	9	8	7	17	16
Mean (SD)	2.8 (2.59)	-2.1 (2.42)	4.4 (2.72)	-1.7 (3.99)	3.5 (2.70)	-1.9 (3.09)
Median	4.0	-1.0	5.0	-1.0	4.0	-1.0
Min, Max	0, 7	-7, 0	1, 8	-7, 5	0, 8	-7, 5
Week 72						
n	6	6	7	7	13	13
Mean (SD)	3.2 (2.64)	-1.3 (3.14)	4.6 (2.64)	-1.6 (2.44)	3.9 (2.63)	-1.5 (2.67)
Median	4.0	0.0	3.0	-1.0	3.0	-1.0
Min, Max	0, 6	-7, 1	2, 8	-4, 3	0, 8	-7, 3

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Table GERMANY.2.5.5.4
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Region
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	3	3	9	9
Mean (SD)	1.3 (1.97)	-3.0 (2.90)	3.0 (1.00)	-4.3 (1.53)	1.9 (1.83)	-3.4 (2.51)
Median	0.5	-4.0	3.0	-4.0	2.0	-4.0
Min, Max	0, 5	-6, 1	2, 4	-6, -3	0, 5	-6, 1
Week 104						
n	1	1	2	2	3	3
Mean (SD)	5.0 (NA)	1.0 (NA)	3.0 (0.00)	-4.0 (1.41)	3.7 (1.15)	-2.3 (3.06)
Median	5.0	1.0	3.0	-4.0	3.0	-3.0
Min, Max	5, 5	1, 1	3, 3	-5, -3	3, 5	-5, 1

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Table GERMANY.2.5.5.4
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Region
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	4.1 (2.44)		4.3 (3.11)		4.2 (2.61)	
Median	4.0		3.5		4.0	
Min, Max	0, 9		0, 9		0, 9	
Week 2						
n	31	31	12	11		
Mean (SD)	3.3 (2.37)	-0.7 (2.50)	4.7 (2.99)	0.2 (1.25)		
Median	3.0	0.0	5.0	0.0		
Min, Max	0, 8	-9, 4	0, 8	-1, 3		
Week 4						
n	32	32	12	12		
Mean (SD)	3.0 (2.51)	-1.1 (2.66)	3.8 (2.60)	-0.6 (2.02)		
Median	2.5	-1.5	3.5	-0.5		
Min, Max	0, 8	-9, 5	0, 8	-5, 3		

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Table GERMANY.2.5.5.4
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by Region
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	26	26	11	11		
Mean (SD)	2.8 (2.44)	-1.0 (2.44)	3.7 (3.20)	-0.5 (2.21)		
Median	2.5	-1.0	3.0	-1.0		
Min, Max	0, 8	-6, 4	0, 8	-5, 4		
Week 12						
n	29	29	12	12		
Mean (SD)	2.4 (2.32)	-1.5 (2.61)	4.2 (3.24)	-0.2 (3.46)		
Median	2.0	-1.0	3.0	-1.0		
Min, Max	0, 8	-9, 3	0, 9	-5, 9		
Week 24						
n	29	29	11	11		
Mean (SD)	2.2 (2.08)	-1.8 (2.12)	2.8 (2.52)	-1.2 (1.89)		
Median	2.0	-2.0	2.0	0.0		
Min, Max	0, 8	-9, 2	0, 9	-6, 0		

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0.
All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported. For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which indicate Anemic symptoms completely prevented patients from doing daily activities.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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ALXN2040-PNH-301

Table GERMANY.2.5.5.4
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Region
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	9	9	36	36
Mean (SD)	2.1 (2.29)	-1.4 (2.29)	1.4 (2.19)	-2.4 (2.88)	1.9 (2.25)	-1.7 (2.45)
Median	1.0	-2.0	1.0	-1.0	1.0	-2.0
Min, Max	0, 7	-6, 4	0, 7	-8, 0	0, 7	-8, 4
Week 56						
n	22	22	6	6	28	28
Mean (SD)	2.4 (2.58)	-1.8 (2.43)	1.2 (0.98)	-2.7 (2.16)	2.1 (2.37)	-2.0 (2.36)
Median	1.5	-2.0	1.0	-2.5	1.0	-2.0
Min, Max	0, 8	-8, 3	0, 3	-6, 0	0, 8	-8, 3
Week 72						
n	12	12	4	4	16	16
Mean (SD)	3.0 (2.73)	-1.3 (2.93)	2.0 (3.37)	-3.0 (2.58)	2.8 (2.82)	-1.7 (2.87)
Median	2.5	-1.5	0.5	-3.0	1.5	-2.0
Min, Max	0, 7	-5, 3	0, 7	-6, 0	0, 7	-6, 3

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:54:17

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Table GERMANY.2.5.5.4
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Region
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	7	3	3	10	10
Mean (SD)	1.7 (1.70)	-3.0 (3.21)	2.0 (1.73)	-2.3 (0.58)	1.8 (1.62)	-2.8 (2.66)
Median	1.0	-1.0	1.0	-2.0	1.0	-2.0
Min, Max	0, 4	-9, 0	1, 4	-3, -2	0, 4	-9, 0
Week 104						
n	2	2	0	0	2	2
Mean (SD)	0.0 (0.00)	-5.0 (5.66)			0.0 (0.00)	-5.0 (5.66)
Median	0.0	-5.0			0.0	-5.0
Min, Max	0, 0	-9, -1			0, 0	-9, -1

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Table GERMANY.2.5.5.5
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		10		34	
Mean (SD)	4.8 (2.40)		5.9 (1.97)		5.1 (2.31)	
Median	4.5		6.0		5.0	
Min, Max	0, 9		2, 9		0, 9	
Week 2						
n	23	23	9	9		
Mean (SD)	3.9 (2.87)	-0.9 (2.91)	5.1 (2.62)	-0.8 (1.56)		
Median	4.0	0.0	6.0	-1.0		
Min, Max	0, 8	-9, 4	2, 8	-3, 2		
Week 4						
n	24	24	10	10		
Mean (SD)	2.8 (2.62)	-1.9 (2.89)	4.7 (1.77)	-1.2 (1.32)		
Median	3.0	-2.0	4.5	-1.0		
Min, Max	0, 7	-9, 4	2, 7	-3, 1		

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0.
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Table GERMANY.2.5.5.5
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	19	19	7	7		
Mean (SD)	3.2 (3.05)	-1.2 (2.50)	6.4 (2.64)	0.6 (1.90)		
Median	4.0	-1.0	8.0	0.0		
Min, Max	0, 8	-5, 4	1, 8	-1, 4		
Week 12						
n	21	21	10	10		
Mean (SD)	2.1 (2.52)	-2.3 (2.56)	5.2 (2.74)	-0.7 (1.77)		
Median	1.0	-2.0	5.0	-1.0		
Min, Max	0, 7	-9, 2	1, 9	-3, 2		
Week 24						
n	19	19	9	9		
Mean (SD)	2.0 (2.52)	-2.5 (2.89)	5.1 (2.52)	-0.8 (1.99)		
Median	1.0	-3.0	5.0	-1.0		
Min, Max	0, 8	-9, 4	2, 9	-3, 2		

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Table GERMANY.2.5.5.5
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	8	8	25	25
Mean (SD)	2.8 (2.88)	-1.2 (2.38)	3.4 (2.50)	-2.3 (2.05)	3.0 (2.72)	-1.5 (2.29)
Median	2.0	-2.0	2.5	-2.0	2.0	-2.0
Min, Max	0, 9	-4, 5	0, 7	-6, 0	0, 9	-6, 5
Week 56						
n	11	11	6	6	17	17
Mean (SD)	2.5 (2.46)	-2.5 (2.25)	3.8 (3.06)	-2.7 (2.66)	2.9 (2.68)	-2.5 (2.32)
Median	2.0	-2.0	4.0	-2.5	2.0	-2.0
Min, Max	0, 7	-8, 0	0, 8	-6, 0	0, 8	-8, 0
Week 72						
n	8	8	5	5	13	13
Mean (SD)	4.3 (2.71)	-0.5 (3.12)	3.8 (3.11)	-2.8 (2.39)	4.1 (2.75)	-1.4 (2.99)
Median	5.0	0.5	3.0	-3.0	5.0	-1.0
Min, Max	0, 7	-5, 3	0, 7	-6, 0	0, 7	-6, 3

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Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.5.5
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	3	3	9	9
Mean (SD)	1.3 (1.97)	-4.2 (3.25)	3.7 (0.58)	-3.0 (1.00)	2.1 (1.96)	-3.8 (2.68)
Median	0.5	-4.5	4.0	-3.0	2.0	-4.0
Min, Max	0, 5	-9, 1	3, 4	-4, -2	0, 5	-9, 1
Week 104						
n	2	2	1	1	3	3
Mean (SD)	2.5 (3.54)	-4.0 (7.07)	3.0 (NA)	-3.0 (NA)	2.7 (2.52)	-3.7 (5.03)
Median	2.5	-4.0	3.0	-3.0	3.0	-3.0
Min, Max	0, 5	-9, 1	3, 3	-3, -3	0, 5	-9, 1

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Table GERMANY.2.5.5.5
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	31		14		45	
Mean (SD)	4.0 (2.59)		4.2 (2.78)		4.1 (2.62)	
Median	4.0		3.5		4.0	
Min, Max	0, 9		0, 8		0, 9	
Week 2						
n	28	28	14	13		
Mean (SD)	3.4 (2.08)	-0.6 (1.89)	4.4 (2.79)	0.0 (1.47)		
Median	3.0	0.0	3.5	0.0		
Min, Max	0, 7	-7, 2	0, 8	-3, 3		
Week 4						
n	33	31	14	14		
Mean (SD)	3.3 (2.58)	-0.7 (2.41)	4.0 (2.83)	-0.2 (1.97)		
Median	3.0	0.0	3.5	0.0		
Min, Max	0, 8	-6, 5	0, 8	-5, 3		

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Table GERMANY.2.5.5.5
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	26	24	11	11		
Mean (SD)	2.7 (2.26)	-1.5 (3.22)	3.1 (2.39)	-0.5 (2.34)		
Median	2.5	-1.5	3.0	0.0		
Min, Max	0, 8	-7, 7	0, 7	-5, 4		
Week 12						
n	30	28	14	14		
Mean (SD)	2.9 (2.62)	-1.2 (2.95)	3.9 (2.77)	-0.4 (3.59)		
Median	2.5	-1.0	3.5	-0.5		
Min, Max	0, 8	-7, 5	0, 9	-5, 9		
Week 24						
n	27	27	13	13		
Mean (SD)	2.5 (2.12)	-1.5 (1.91)	2.2 (1.72)	-1.8 (2.52)		
Median	2.0	-2.0	2.0	-1.0		
Min, Max	0, 8	-5, 3	0, 6	-7, 1		

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Table GERMANY.2.5.5.5
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	24	24	10	10	34	34
Mean (SD)	2.4 (2.36)	-1.5 (2.72)	1.2 (1.48)	-3.1 (2.64)	2.1 (2.19)	-2.0 (2.76)
Median	2.0	-2.0	1.0	-3.0	1.0	-2.0
Min, Max	0, 7	-7, 4	0, 5	-8, 0	0, 7	-8, 4
Week 56						
n	20	20	8	7	28	27
Mean (SD)	2.6 (2.65)	-1.6 (2.46)	2.4 (2.33)	-1.7 (3.73)	2.5 (2.52)	-1.6 (2.76)
Median	1.5	-1.5	1.0	-2.0	1.0	-2.0
Min, Max	0, 8	-7, 3	1, 7	-7, 5	0, 8	-7, 5
Week 72						
n	10	10	6	6	16	16
Mean (SD)	2.1 (2.23)	-1.9 (2.73)	3.5 (3.27)	-1.5 (2.59)	2.6 (2.66)	-1.8 (2.59)
Median	1.5	-1.5	2.5	-1.5	2.0	-1.5
Min, Max	0, 7	-7, 2	0, 8	-4, 3	0, 8	-7, 3

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0.
All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported. For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activities.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Change from baseline is summarized only for patients who have data at baseline and the specified time point. Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.5.5
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	7	3	3	10	10
Mean (SD)	1.7 (1.70)	-2.0 (2.45)	1.3 (0.58)	-3.7 (2.08)	1.6 (1.43)	-2.5 (2.37)
Median	1.0	-1.0	1.0	-3.0	1.0	-1.5
Min, Max	0, 4	-6, 0	1, 2	-6, -2	0, 4	-6, 0
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.0 (NA)	-1.0 (NA)	3.0 (NA)	-5.0 (NA)	1.5 (2.12)	-3.0 (2.83)
Median	0.0	-1.0	3.0	-5.0	1.5	-3.0
Min, Max	0, 0	-1, -1	3, 3	-5, -5	0, 3	-5, -1

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0.
All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported. For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which indicate Anemic symptoms completely prevented patients from doing daily activities.
Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.
D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
Change from baseline is summarized only for patients who have data at baseline and the specified time point. Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).
Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.5.6
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		15		48	
Mean (SD)	4.4 (2.15)		5.3 (2.63)		4.6 (2.32)	
Median	4.0		6.0		5.0	
Min, Max	0, 9		0, 9		0, 9	
Week 2						
n	31	31	14	14		
Mean (SD)	3.9 (2.52)	-0.4 (1.76)	4.9 (2.81)	-0.4 (1.60)		
Median	4.0	0.0	6.0	0.0		
Min, Max	0, 8	-5, 2	0, 8	-3, 3		
Week 4						
n	35	33	15	15		
Mean (SD)	3.2 (2.49)	-1.2 (2.29)	4.4 (2.53)	-0.9 (2.10)		
Median	4.0	-1.0	4.0	-1.0		
Min, Max	0, 8	-5, 4	0, 8	-5, 3		

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
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Table GERMANY.2.5.5.6

Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	29	27	11	11		
Mean (SD)	3.6 (2.71)	-0.6 (2.78)	4.4 (3.26)	-0.7 (2.28)		
Median	4.0	-1.0	4.0	-1.0		
Min, Max	0, 8	-5, 7	0, 8	-5, 4		
Week 12						
n	31	29	15	15		
Mean (SD)	2.7 (2.31)	-1.5 (2.25)	5.1 (2.96)	-0.2 (3.21)		
Median	2.0	-2.0	5.0	-1.0		
Min, Max	0, 8	-6, 3	0, 9	-5, 9		
Week 24						
n	27	27	13	13		
Mean (SD)	2.4 (2.22)	-1.7 (2.10)	3.6 (2.93)	-1.4 (2.40)		
Median	2.0	-2.0	3.0	-1.0		
Min, Max	0, 8	-6, 4	0, 9	-7, 2		

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Table GERMANY.2.5.5.6

Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	25	25	11	11	36	36
Mean (SD)	2.9 (2.76)	-1.0 (2.44)	2.7 (2.37)	-2.5 (2.07)	2.8 (2.61)	-1.4 (2.41)
Median	2.0	-2.0	2.0	-2.0	2.0	-2.0
Min, Max	0, 9	-5, 5	0, 7	-7, 0	0, 9	-7, 5
Week 56						
n	19	19	9	9	28	28
Mean (SD)	3.0 (2.81)	-1.1 (1.87)	3.1 (2.62)	-2.0 (2.00)	3.0 (2.70)	-1.4 (1.93)
Median	2.0	-1.0	2.0	-1.0	2.0	-1.0
Min, Max	0, 8	-4, 3	1, 8	-5, 0	0, 8	-5, 3
Week 72						
n	10	10	7	7	17	17
Mean (SD)	2.8 (2.74)	-1.3 (2.45)	4.3 (3.04)	-1.9 (2.67)	3.4 (2.87)	-1.5 (2.48)
Median	2.5	-1.5	3.0	-3.0	3.0	-2.0
Min, Max	0, 7	-5, 3	0, 8	-4, 3	0, 8	-5, 3

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0.

All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported. For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activities.

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.5.6

Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	7	3	3	10	10
Mean (SD)	1.9 (1.68)	-2.1 (1.86)	2.7 (1.53)	-3.3 (0.58)	2.1 (1.60)	-2.5 (1.65)
Median	2.0	-1.0	3.0	-3.0	2.0	-3.0
Min, Max	0, 4	-5, 0	1, 4	-4, -3	0, 4	-5, 0
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.0 (NA)	-1.0 (NA)	3.0 (NA)	-3.0 (NA)	1.5 (2.12)	-2.0 (1.41)
Median	0.0	-1.0	3.0	-3.0	1.5	-2.0
Min, Max	0, 0	-1, -1	3, 3	-3, -3	0, 3	-3, -1

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Table GERMANY.2.5.5.6

Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	4.3 (3.04)		4.3 (2.50)		4.3 (2.85)	
Median	4.5		3.0		4.0	
Min, Max	0, 9		2, 8		0, 9	
Week 2						
n	20	20	9	8		
Mean (SD)	3.1 (2.31)	-1.3 (3.08)	4.3 (2.60)	-0.3 (1.49)		
Median	3.0	0.0	3.0	0.0		
Min, Max	0, 7	-9, 4	2, 8	-3, 2		
Week 4						
n	22	22	9	9		
Mean (SD)	2.9 (2.76)	-1.4 (3.22)	4.1 (2.37)	-0.2 (0.97)		
Median	2.0	-1.5	4.0	0.0		
Min, Max	0, 8	-9, 5	2, 8	-1, 2		

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Table GERMANY.2.5.5.6

Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	16	16	7	7		
Mean (SD)	1.7 (1.96)	-2.5 (2.78)	4.4 (2.57)	0.9 (1.77)		
Median	1.0	-2.5	4.0	1.0		
Min, Max	0, 7	-7, 2	1, 7	-1, 4		
Week 12						
n	20	20	9	9		
Mean (SD)	2.4 (3.00)	-2.0 (3.52)	3.3 (2.18)	-1.0 (2.45)		
Median	1.0	-1.5	3.0	-1.0		
Min, Max	0, 8	-9, 5	0, 7	-5, 2		
Week 24						
n	19	19	9	9		
Mean (SD)	2.1 (2.39)	-2.2 (2.77)	3.0 (1.87)	-1.3 (2.35)		
Median	1.0	-2.0	2.0	-1.0		
Min, Max	0, 8	-9, 3	1, 7	-6, 2		

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All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported. For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which indicate Anemic symptoms completely prevented patients from doing daily activities.

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point. Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.5.6

Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	16	16	7	7	23	23
Mean (SD)	2.1 (2.22)	-2.0 (2.68)	1.3 (1.80)	-3.1 (2.91)	1.9 (2.10)	-2.3 (2.74)
Median	1.5	-2.5	1.0	-3.0	1.0	-3.0
Min, Max	0, 6	-7, 4	0, 5	-8, 0	0, 6	-8, 4
Week 56						
n	12	12	5	4	17	16
Mean (SD)	1.8 (1.91)	-3.2 (2.62)	2.8 (3.03)	-2.5 (5.45)	2.1 (2.25)	-3.0 (3.33)
Median	1.0	-3.0	1.0	-4.0	1.0	-3.0
Min, Max	0, 5	-8, 0	0, 7	-7, 5	0, 7	-8, 5
Week 72						
n	8	8	4	4	12	12
Mean (SD)	3.4 (2.62)	-1.3 (3.58)	2.5 (3.11)	-2.5 (2.38)	3.1 (2.68)	-1.7 (3.17)
Median	4.5	0.5	1.5	-1.5	3.0	-1.0
Min, Max	0, 6	-7, 3	0, 7	-6, -1	0, 7	-7, 3

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Table GERMANY.2.5.5.6

Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	3	3	9	9
Mean (SD)	1.2 (1.94)	-4.0 (3.79)	2.3 (1.53)	-3.3 (2.31)	1.6 (1.81)	-3.8 (3.23)
Median	0.5	-5.0	2.0	-2.0	1.0	-5.0
Min, Max	0, 5	-9, 1	1, 4	-6, -2	0, 5	-9, 1
Week 104						
n	2	2	1	1	3	3
Mean (SD)	2.5 (3.54)	-4.0 (7.07)	3.0 (NA)	-5.0 (NA)	2.7 (2.52)	-4.3 (5.03)
Median	2.5	-4.0	3.0	-5.0	3.0	-5.0
Min, Max	0, 5	-9, 1	3, 3	-5, -5	0, 5	-9, 1

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.5.7
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	20		10		30	
Mean (SD)	4.1 (2.65)		5.4 (1.17)		4.5 (2.33)	
Median	4.5		6.0		5.0	
Min, Max	0, 9		3, 7		0, 9	
Week 2						
n	19	19	9	8		
Mean (SD)	3.8 (2.57)	-0.5 (2.09)	5.1 (2.57)	-0.6 (2.33)		
Median	3.0	0.0	6.0	-0.5		
Min, Max	0, 8	-5, 2	2, 8	-3, 3		
Week 4						
n	21	20	10	10		
Mean (SD)	3.2 (2.43)	-1.0 (2.28)	5.0 (2.16)	-0.4 (1.51)		
Median	4.0	-0.5	4.5	-0.5		
Min, Max	0, 7	-5, 4	2, 8	-3, 2		

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.
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How much anemic symptoms affect ability on regular daily activities, other than work
Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	14	13	5	5		
Mean (SD)	3.3 (2.95)	-0.8 (3.32)	4.6 (1.52)	-0.4 (1.95)		
Median	3.5	0.0	4.0	-1.0		
Min, Max	0, 8	-5, 7	3, 7	-3, 2		
Week 12						
n	17	16	10	10		
Mean (SD)	3.1 (3.07)	-1.1 (2.99)	4.6 (2.27)	-0.8 (2.20)		
Median	2.0	-1.0	4.5	-1.0		
Min, Max	0, 8	-6, 5	0, 7	-3, 2		
Week 24						
n	12	12	9	9		
Mean (SD)	2.9 (3.15)	-0.7 (2.77)	3.8 (2.64)	-1.6 (3.05)		
Median	2.5	-1.0	3.0	-2.0		
Min, Max	0, 8	-6, 4	0, 8	-7, 2		

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How much anemic symptoms affect ability on regular daily activities, other than work
Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	14	14	7	7	21	21
Mean (SD)	3.2 (2.99)	-1.1 (2.80)	1.7 (1.50)	-4.1 (1.77)	2.7 (2.65)	-2.1 (2.85)
Median	2.5	-2.0	2.0	-4.0	2.0	-3.0
Min, Max	0, 9	-6, 5	0, 4	-7, -2	0, 9	-7, 5
Week 56						
n	9	9	7	6	16	15
Mean (SD)	2.7 (2.83)	-1.6 (1.67)	2.9 (2.41)	-3.2 (2.32)	2.8 (2.57)	-2.2 (2.04)
Median	1.0	-1.0	2.0	-3.5	1.5	-2.0
Min, Max	0, 7	-5, 0	0, 6	-6, 0	0, 7	-6, 0
Week 72						
n	5	5	7	7	12	12
Mean (SD)	1.0 (1.22)	-2.4 (1.95)	2.6 (2.70)	-2.7 (2.93)	1.9 (2.27)	-2.6 (2.47)
Median	1.0	-3.0	2.0	-4.0	1.5	-3.0
Min, Max	0, 3	-5, 0	0, 8	-6, 3	0, 8	-6, 3

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Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	5	5	3	3	8	8
Mean (SD)	1.0 (1.73)	-2.2 (2.59)	2.7 (1.53)	-2.7 (0.58)	1.6 (1.77)	-2.4 (2.00)
Median	0.0	-1.0	3.0	-3.0	1.0	-2.5
Min, Max	0, 4	-5, 0	1, 4	-3, -2	0, 4	-5, 0
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.0 (NA)	-1.0 (NA)	3.0 (NA)	-3.0 (NA)	1.5 (2.12)	-2.0 (1.41)
Median	0.0	-1.0	3.0	-3.0	1.5	-2.0
Min, Max	0, 0	-1, -1	3, 3	-3, -3	0, 3	-3, -1

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How much anemic symptoms affect ability on regular daily activities, other than work
Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		14		49	
Mean (SD)	4.5 (2.47)		4.6 (3.23)		4.5 (2.67)	
Median	4.0		3.5		4.0	
Min, Max	0, 9		0, 9		0, 9	
Week 2						
n	32	32	14	14		
Mean (SD)	3.5 (2.41)	-0.9 (2.56)	4.4 (2.82)	-0.1 (0.86)		
Median	3.0	0.0	3.5	0.0		
Min, Max	0, 8	-9, 4	0, 8	-1, 2		
Week 4						
n	36	35	14	14		
Mean (SD)	3.0 (2.69)	-1.4 (2.89)	3.8 (2.55)	-0.8 (1.97)		
Median	2.0	-2.0	3.5	-1.0		
Min, Max	0, 8	-9, 5	0, 8	-5, 3		

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How much anemic symptoms affect ability on regular daily activities, other than work
Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	31	30	13	13		
Mean (SD)	2.7 (2.47)	-1.6 (2.71)	4.3 (3.38)	0.0 (2.35)		
Median	2.0	-1.5	3.0	0.0		
Min, Max	0, 8	-7, 4	0, 8	-5, 4		
Week 12						
n	34	33	14	14		
Mean (SD)	2.3 (2.31)	-2.0 (2.73)	4.3 (3.17)	-0.3 (3.41)		
Median	2.0	-2.0	3.0	-1.0		
Min, Max	0, 8	-9, 3	0, 9	-5, 9		
Week 24						
n	34	34	13	13		
Mean (SD)	2.1 (1.89)	-2.4 (2.10)	3.1 (2.50)	-1.2 (1.79)		
Median	2.0	-2.0	2.0	-1.0		
Min, Max	0, 8	-9, 2	0, 9	-6, 0		

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How much anemic symptoms affect ability on regular daily activities, other than work
Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	11	11	38	38
Mean (SD)	2.3 (2.30)	-1.5 (2.47)	2.5 (2.62)	-1.8 (2.32)	2.3 (2.36)	-1.6 (2.40)
Median	2.0	-2.0	1.0	-1.0	1.5	-1.5
Min, Max	0, 7	-7, 4	0, 7	-8, 0	0, 7	-8, 4
Week 56						
n	22	22	7	7	29	29
Mean (SD)	2.5 (2.48)	-2.0 (2.65)	3.1 (3.08)	-1.3 (3.73)	2.6 (2.60)	-1.8 (2.89)
Median	2.0	-2.0	1.0	-1.0	2.0	-2.0
Min, Max	0, 8	-8, 3	1, 8	-7, 5	0, 8	-8, 5
Week 72						
n	13	13	4	4	17	17
Mean (SD)	3.8 (2.61)	-0.8 (3.16)	5.5 (3.00)	-1.0 (0.82)	4.2 (2.70)	-0.9 (2.76)
Median	5.0	0.0	7.0	-1.0	5.0	-1.0
Min, Max	0, 7	-7, 3	1, 7	-2, 0	0, 7	-7, 3

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Table GERMANY.2.5.5.7

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How much anemic symptoms affect ability on regular daily activities, other than work
Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	8	8	3	3	11	11
Mean (SD)	1.9 (1.81)	-3.5 (3.21)	2.3 (1.53)	-4.0 (2.00)	2.0 (1.67)	-3.6 (2.84)
Median	1.5	-3.5	2.0	-4.0	2.0	-4.0
Min, Max	0, 5	-9, 1	1, 4	-6, -2	0, 5	-9, 1
Week 104						
n	2	2	1	1	3	3
Mean (SD)	2.5 (3.54)	-4.0 (7.07)	3.0 (NA)	-5.0 (NA)	2.7 (2.52)	-4.3 (5.03)
Median	2.5	-4.0	3.0	-5.0	3.0	-5.0
Min, Max	0, 5	-9, 1	3, 3	-5, -5	0, 5	-9, 1

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Table GERMANY.2.5.5.8

Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	4.3 (2.49)		1.3 (0.96)		3.3 (2.53)	
Median	3.5		1.5		2.5	
Min, Max	1, 8		0, 2		0, 8	
Week 2						
n	8	8	4	4		
Mean (SD)	3.3 (2.60)	-1.0 (1.85)	1.8 (1.71)	0.5 (1.00)		
Median	3.0	-1.5	1.5	0.0		
Min, Max	0, 7	-3, 2	0, 4	0, 2		
Week 4						
n	8	8	4	4		
Mean (SD)	2.6 (1.92)	-1.6 (1.85)	2.5 (1.91)	1.3 (1.50)		
Median	2.5	-1.5	3.0	1.0		
Min, Max	0, 5	-5, 1	0, 4	0, 3		

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Table GERMANY.2.5.5.8

Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	8	8	4	4		
Mean (SD)	2.4 (2.88)	-1.9 (2.75)	1.3 (1.26)	0.0 (0.82)		
Median	1.5	-2.5	1.0	0.0		
Min, Max	0, 8	-5, 4	0, 3	-1, 1		
Week 12						
n	8	8	4	4		
Mean (SD)	2.0 (2.33)	-2.3 (2.31)	3.3 (4.03)	2.0 (4.76)		
Median	1.5	-1.5	2.0	0.0		
Min, Max	0, 6	-6, 1	0, 9	-1, 9		
Week 24						
n	8	8	4	4		
Mean (SD)	2.8 (2.71)	-1.5 (1.85)	1.3 (0.96)	0.0 (0.00)		
Median	2.5	-1.0	1.5	0.0		
Min, Max	0, 8	-4, 2	0, 2	0, 0		

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How much anemic symptoms affect ability on regular daily activities, other than work
Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	1.2 (1.60)	-2.5 (1.38)	1.3 (0.58)	-0.3 (0.58)	1.2 (1.30)	-1.8 (1.56)
Median	0.5	-2.0	1.0	0.0	1.0	-2.0
Min, Max	0, 4	-5, -1	1, 2	-1, 0	0, 4	-5, 0
Week 56						
n	4	4	1	1	5	5
Mean (SD)	1.5 (3.00)	-1.8 (1.50)	1.0 (NA)	0.0 (NA)	1.4 (2.61)	-1.4 (1.52)
Median	0.0	-2.0	1.0	0.0	0.0	-1.0
Min, Max	0, 6	-3, 0	1, 1	0, 0	0, 6	-3, 0
Week 72						
n	1	1	0	0	1	1
Mean (SD)	0.0 (NA)	-3.0 (NA)			0.0 (NA)	-3.0 (NA)
Median	0.0	-3.0			0.0	-3.0
Min, Max	0, 0	-3, -3			0, 0	-3, -3

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How much anemic symptoms affect ability on regular daily activities, other than work
Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	47		20		67	
Mean (SD)	4.3 (2.55)		5.7 (2.11)		4.7 (2.48)	
Median	5.0		6.0		5.0	
Min, Max	0, 9		2, 9		0, 9	
Week 2						
n	43	43	19	18		
Mean (SD)	3.7 (2.45)	-0.7 (2.48)	5.3 (2.45)	-0.5 (1.58)		
Median	3.0	0.0	6.0	-0.5		
Min, Max	0, 8	-9, 4	2, 8	-3, 3		
Week 4						
n	49	47	20	20		
Mean (SD)	3.2 (2.68)	-1.2 (2.80)	4.7 (2.39)	-1.0 (1.59)		
Median	3.0	-1.0	4.5	-1.0		
Min, Max	0, 8	-9, 5	0, 8	-5, 2		

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How much anemic symptoms affect ability on regular daily activities, other than work
Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	37	35	14	14		
Mean (SD)	3.0 (2.57)	-1.2 (2.95)	5.3 (2.64)	-0.1 (2.48)		
Median	3.0	-1.0	6.0	-0.5		
Min, Max	0, 8	-7, 7	0, 8	-5, 4		
Week 12						
n	43	41	20	20		
Mean (SD)	2.7 (2.63)	-1.6 (2.92)	4.7 (2.54)	-1.0 (2.27)		
Median	2.0	-2.0	4.0	-1.0		
Min, Max	0, 8	-9, 5	0, 9	-5, 2		
Week 24						
n	38	38	18	18		
Mean (SD)	2.2 (2.20)	-2.0 (2.49)	3.8 (2.53)	-1.7 (2.47)		
Median	2.0	-2.0	3.0	-1.0		
Min, Max	0, 8	-9, 4	0, 9	-7, 2		

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How much anemic symptoms affect ability on regular daily activities, other than work
Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	35	35	15	15	50	50
Mean (SD)	2.8 (2.63)	-1.2 (2.67)	2.3 (2.41)	-3.2 (2.31)	2.7 (2.55)	-1.8 (2.71)
Median	2.0	-2.0	2.0	-3.0	2.0	-2.0
Min, Max	0, 9	-7, 5	0, 7	-8, 0	0, 9	-8, 5
Week 56						
n	27	27	13	12	40	39
Mean (SD)	2.7 (2.50)	-1.9 (2.52)	3.2 (2.70)	-2.3 (3.26)	2.8 (2.54)	-2.0 (2.73)
Median	2.0	-2.0	2.0	-2.5	2.0	-2.0
Min, Max	0, 8	-8, 3	0, 8	-7, 5	0, 8	-8, 5
Week 72						
n	17	17	11	11	28	28
Mean (SD)	3.2 (2.59)	-1.2 (2.96)	3.6 (3.04)	-2.1 (2.47)	3.4 (2.73)	-1.5 (2.77)
Median	3.0	-1.0	3.0	-2.0	3.0	-1.0
Min, Max	0, 7	-7, 3	0, 8	-6, 3	0, 8	-7, 3

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How much anemic symptoms affect ability on regular daily activities, other than work
Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	13	13	6	6	19	19
Mean (SD)	1.5 (1.76)	-3.0 (2.94)	2.5 (1.38)	-3.3 (1.51)	1.8 (1.68)	-3.1 (2.54)
Median	1.0	-3.0	2.5	-3.0	1.0	-3.0
Min, Max	0, 5	-9, 1	1, 4	-6, -2	0, 5	-9, 1
Week 104						
n	3	3	2	2	5	5
Mean (SD)	1.7 (2.89)	-3.0 (5.29)	3.0 (0.00)	-4.0 (1.41)	2.2 (2.17)	-3.4 (3.85)
Median	0.0	-1.0	3.0	-4.0	3.0	-3.0
Min, Max	0, 5	-9, 1	3, 3	-5, -3	0, 5	-9, 1

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Table GERMANY.2.5.5.9
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Race
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	3.5 (2.11)		3.8 (3.12)		3.6 (2.42)	
Median	3.5		2.5		3.0	
Min, Max	0, 8		0, 9		0, 9	
Week 2						
n	21	21	10	10		
Mean (SD)	3.1 (2.17)	-0.4 (1.47)	4.0 (2.83)	0.2 (1.32)		
Median	3.0	0.0	3.5	0.0		
Min, Max	0, 7	-3, 2	0, 8	-1, 3		
Week 4						
n	22	22	10	10		
Mean (SD)	2.4 (2.11)	-1.2 (2.26)	3.1 (2.28)	-0.7 (2.21)		
Median	1.5	-1.5	3.0	-1.0		
Min, Max	0, 6	-5, 5	0, 7	-5, 3		

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Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	18	18	10	10		
Mean (SD)	2.4 (2.36)	-1.1 (2.29)	3.4 (3.17)	-0.4 (2.32)		
Median	2.0	-1.0	3.0	-0.5		
Min, Max	0, 8	-5, 4	0, 8	-5, 4		
Week 12						
n	20	20	10	10		
Mean (SD)	2.0 (1.95)	-1.4 (2.14)	4.1 (3.51)	0.3 (3.62)		
Median	2.0	-1.0	3.0	-0.5		
Min, Max	0, 6	-6, 3	0, 9	-5, 9		
Week 24						
n	20	20	9	9		
Mean (SD)	2.1 (2.13)	-1.3 (1.72)	2.9 (2.80)	-0.4 (0.73)		
Median	1.5	-1.0	2.0	0.0		
Min, Max	0, 8	-4, 2	0, 9	-2, 0		

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How much anemic symptoms affect ability on regular daily activities, other than work
Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	7	7	27	27
Mean (SD)	1.7 (1.90)	-1.7 (2.28)	1.9 (2.34)	-1.1 (1.35)	1.7 (1.98)	-1.5 (2.06)
Median	1.0	-2.0	1.0	-1.0	1.0	-2.0
Min, Max	0, 6	-6, 4	0, 7	-3, 0	0, 7	-6, 4
Week 56						
n	16	16	5	5	21	21
Mean (SD)	1.9 (2.36)	-1.7 (1.82)	1.4 (0.89)	-2.0 (1.58)	1.8 (2.10)	-1.8 (1.73)
Median	1.0	-2.0	1.0	-2.0	1.0	-2.0
Min, Max	0, 7	-5, 2	1, 3	-4, 0	0, 7	-5, 2
Week 72						
n	8	8	3	3	11	11
Mean (SD)	2.3 (3.01)	-1.6 (2.97)	2.7 (3.79)	-2.0 (2.00)	2.4 (3.04)	-1.7 (2.65)
Median	1.0	-2.0	1.0	-2.0	1.0	-2.0
Min, Max	0, 7	-5, 3	0, 7	-4, 0	0, 7	-5, 3

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Table GERMANY.2.5.5.9
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Race
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	2	2	8	8
Mean (SD)	2.0 (1.67)	-2.0 (2.00)	1.0 (0.00)	-2.5 (0.71)	1.8 (1.49)	-2.1 (1.73)
Median	1.5	-1.0	1.0	-2.5	1.0	-1.5
Min, Max	0, 4	-5, 0	1, 1	-3, -2	0, 4	-5, 0
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.0 (NA)	-1.0 (NA)			0.0 (NA)	-1.0 (NA)
Median	0.0	-1.0			0.0	-1.0
Min, Max	0, 0	-1, -1			0, 0	-1, -1

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Table GERMANY.2.5.5.9
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Race
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	26		10		36	
Mean (SD)	5.0 (2.74)		5.6 (2.01)		5.2 (2.55)	
Median	5.0		6.0		6.0	
Min, Max	0, 9		2, 8		0, 9	
Week 2						
n	23	23	10	9		
Mean (SD)	4.0 (2.77)	-1.2 (3.21)	5.1 (2.51)	-0.8 (1.39)		
Median	3.0	0.0	4.5	0.0		
Min, Max	0, 8	-9, 4	2, 8	-3, 1		
Week 4						
n	28	26	10	10		
Mean (SD)	3.2 (2.63)	-1.8 (2.98)	4.9 (2.47)	-0.7 (1.64)		
Median	3.5	-2.0	4.5	-0.5		
Min, Max	0, 7	-9, 4	2, 8	-3, 2		

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Table GERMANY.2.5.5.9
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Race
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	22	20	7	7		
Mean (SD)	3.2 (2.81)	-1.7 (3.44)	5.4 (2.30)	0.0 (2.16)		
Median	3.0	-1.5	5.0	0.0		
Min, Max	0, 8	-7, 7	2, 8	-3, 4		
Week 12						
n	24	22	10	10		
Mean (SD)	3.0 (2.71)	-2.0 (3.42)	4.4 (2.37)	-1.2 (1.87)		
Median	3.0	-2.0	4.0	-1.5		
Min, Max	0, 8	-9, 5	0, 8	-3, 2		
Week 24						
n	19	19	9	9		
Mean (SD)	2.4 (2.34)	-2.6 (3.06)	2.4 (1.42)	-3.1 (2.32)		
Median	2.0	-3.0	3.0	-3.0		
Min, Max	0, 8	-9, 4	0, 5	-7, 0		

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Table GERMANY.2.5.5.9
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by Race
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	14	14	9	9	23	23
Mean (SD)	3.4 (2.90)	-1.3 (3.15)	2.1 (2.32)	-3.8 (2.64)	2.9 (2.71)	-2.3 (3.15)
Median	2.5	-1.5	2.0	-3.0	2.0	-2.0
Min, Max	0, 9	-7, 5	0, 7	-8, -1	0, 9	-8, 5
Week 56						
n	10	10	6	6	16	16
Mean (SD)	2.5 (2.46)	-2.9 (2.81)	3.8 (3.31)	-1.8 (4.07)	3.0 (2.78)	-2.5 (3.25)
Median	2.0	-2.0	3.5	-2.5	2.5	-2.0
Min, Max	0, 7	-8, 0	0, 8	-6, 5	0, 8	-8, 5
Week 72						
n	7	7	6	6	13	13
Mean (SD)	3.6 (2.64)	-1.4 (3.41)	2.8 (2.32)	-3.2 (1.94)	3.2 (2.42)	-2.2 (2.86)
Median	5.0	0.0	2.5	-3.5	3.0	-3.0
Min, Max	0, 6	-7, 3	0, 7	-6, -1	0, 7	-7, 3

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Table GERMANY.2.5.5.9
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Race
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	3	3	9	9
Mean (SD)	0.5 (0.84)	-4.7 (3.01)	3.7 (0.58)	-3.0 (1.00)	1.6 (1.74)	-4.1 (2.57)
Median	0.0	-5.0	4.0	-3.0	1.0	-4.0
Min, Max	0, 2	-9, 0	3, 4	-4, -2	0, 4	-9, 0
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.0 (NA)	-9.0 (NA)	3.0 (NA)	-3.0 (NA)	1.5 (2.12)	-6.0 (4.24)
Median	0.0	-9.0	3.0	-3.0	1.5	-6.0
Min, Max	0, 0	-9, -9	3, 3	-3, -3	0, 3	-9, -3

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by Race
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		4		11	
Mean (SD)	4.3 (2.43)		6.0 (1.41)		4.9 (2.21)	
Median	5.0		5.5		5.0	
Min, Max	0, 8		5, 8		0, 8	
Week 2						
n	7	7	3	3		
Mean (SD)	3.9 (2.19)	-0.4 (0.98)	5.7 (3.21)	-0.7 (2.52)		
Median	4.0	0.0	7.0	-1.0		
Min, Max	0, 7	-2, 1	2, 8	-3, 2		
Week 4						
n	7	7	4	4		
Mean (SD)	5.0 (3.06)	0.7 (1.89)	5.8 (1.50)	-0.3 (0.96)		
Median	6.0	1.0	6.0	-0.5		
Min, Max	1, 8	-2, 3	4, 7	-1, 1		

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by Race
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	5	5	1	1		
Mean (SD)	3.2 (2.86)	-1.0 (2.92)	7.0 (NA)	2.0 (NA)		
Median	3.0	0.0	7.0	2.0		
Min, Max	0, 7	-5, 2	7, 7	2, 2		
Week 12						
n	7	7	4	4		
Mean (SD)	2.9 (3.67)	-1.4 (2.70)	5.3 (2.06)	-0.8 (3.40)		
Median	1.0	-2.0	5.5	0.0		
Min, Max	0, 8	-5, 3	3, 7	-5, 2		
Week 24						
n	7	7	4	4		
Mean (SD)	2.4 (2.82)	-1.9 (1.57)	6.5 (1.29)	0.5 (2.38)		
Median	2.0	-2.0	6.5	1.5		
Min, Max	0, 8	-4, 0	5, 8	-3, 2		

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Table GERMANY.2.5.5.9
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by Race
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	7	7	2	2	9	9
Mean (SD)	3.6 (2.88)	-0.7 (2.21)	3.5 (2.12)	-3.5 (0.71)	3.6 (2.60)	-1.3 (2.29)
Median	5.0	0.0	3.5	-3.5	5.0	-3.0
Min, Max	0, 7	-3, 2	2, 5	-4, -3	0, 7	-4, 2
Week 56						
n	5	5	3	2	8	7
Mean (SD)	4.6 (2.61)	-0.4 (2.70)	4.0 (2.65)	-3.5 (4.95)	4.4 (2.45)	-1.3 (3.35)
Median	4.0	0.0	5.0	-3.5	4.5	0.0
Min, Max	1, 8	-4, 3	1, 6	-7, 0	1, 8	-7, 3
Week 72						
n	3	3	2	2	5	5
Mean (SD)	4.0 (1.00)	0.0 (1.73)	7.5 (0.71)	1.0 (2.83)	5.4 (2.07)	0.4 (1.95)
Median	4.0	1.0	7.5	1.0	5.0	1.0
Min, Max	3, 5	-2, 1	7, 8	-1, 3	3, 8	-2, 3

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Table GERMANY.2.5.5.9
Summary of Actual Values and Change from Baseline in WPAI:ANS During Treatment Period 1 and Period 2 and Long Term Extension
by Race
Modified Randomized Set

How much anemic symptoms affect ability on regular daily activities, other than work
Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	1	1	1	1	2	2
Mean (SD)	5.0 (NA)	1.0 (NA)	2.0 (NA)	-6.0 (NA)	3.5 (2.12)	-2.5 (4.95)
Median	5.0	1.0	2.0	-6.0	3.5	-2.5
Min, Max	5, 5	1, 1	2, 2	-6, -6	2, 5	-6, 1
Week 104						
n	1	1	1	1	2	2
Mean (SD)	5.0 (NA)	1.0 (NA)	3.0 (NA)	-5.0 (NA)	4.0 (1.41)	-2.0 (4.24)
Median	5.0	1.0	3.0	-5.0	4.0	-2.0
Min, Max	5, 5	1, 1	3, 3	-5, -5	3, 5	-5, 1

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Table GERMANY.2.5.9.2
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.47 (0.381)	-0.19 (0.628)	-0.28 (0.720)	0.7007
	95% CI for LS MEAN	(-1.25, 0.31)	(-1.48, 1.10)	(-1.76, 1.20)	
Week 4	LS Mean (SEM)	-0.82 (0.480)	-0.31 (0.744)	-0.51 (0.878)	0.5687
	95% CI for LS MEAN	(-1.80, 0.16)	(-1.83, 1.21)	(-2.30, 1.29)	
Week 8	LS Mean (SEM)	-1.27 (0.589)	-0.12 (0.940)	-1.15 (1.105)	0.3048
	95% CI for LS MEAN	(-2.48, -0.07)	(-2.04, 1.81)	(-3.42, 1.11)	
Week 12	LS Mean (SEM)	-1.61 (0.573)	0.47 (0.870)	-2.08 (1.036)	0.0543
	95% CI for LS MEAN	(-2.78, -0.44)	(-1.31, 2.25)	(-4.20, 0.04)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.9.2
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Female

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.37 (0.412)	-0.13 (0.598)	-1.25 (0.731)	0.0956
	95% CI for LS MEAN	(-2.21, -0.54)	(-1.34, 1.08)	(-2.72, 0.23)	
Week 4	LS Mean (SEM)	-1.82 (0.393)	-0.58 (0.585)	-1.24 (0.708)	0.0860
	95% CI for LS MEAN	(-2.62, -1.03)	(-1.76, 0.60)	(-2.67, 0.18)	
Week 8	LS Mean (SEM)	-1.93 (0.464)	0.14 (0.687)	-2.06 (0.830)	0.0180
	95% CI for LS MEAN	(-2.87, -0.98)	(-1.26, 1.53)	(-3.75, -0.38)	
Week 12	LS Mean (SEM)	-1.86 (0.449)	-0.85 (0.645)	-1.01 (0.788)	0.2061
	95% CI for LS MEAN	(-2.76, -0.95)	(-2.15, 0.46)	(-2.60, 0.58)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.9.2
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.5905
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.9.3
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by Age at Informed Consent
(< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

< 65 Years

Visit	Statistics	Danicopan (N = 40)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.21 (0.340)	-0.19 (0.500)	-1.03 (0.598)	0.0915
	95% CI for LS MEAN	(-1.89, -0.53)	(-1.19, 0.82)	(-2.23, 0.17)	
Week 4	LS Mean (SEM)	-1.57 (0.372)	-0.80 (0.558)	-0.77 (0.664)	0.2493
	95% CI for LS MEAN	(-2.32, -0.82)	(-1.91, 0.32)	(-2.10, 0.56)	
Week 8	LS Mean (SEM)	-2.10 (0.388)	0.10 (0.579)	-2.20 (0.692)	0.0026
	95% CI for LS MEAN	(-2.88, -1.32)	(-1.07, 1.27)	(-3.60, -0.81)	
Week 12	LS Mean (SEM)	-1.99 (0.396)	-0.74 (0.584)	-1.24 (0.700)	0.0806
	95% CI for LS MEAN	(-2.78, -1.19)	(-1.91, 0.43)	(-2.65, 0.16)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.9.3
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by Age at Informed Consent
(< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

≥ 65 Years

Visit	Statistics	Danicopan (N = 15)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.11 (0.483)	-0.49 (0.892)	0.38 (1.036)	0.7160
	95% CI for LS MEAN	(-1.14, 0.92)	(-2.38, 1.39)	(-1.80, 2.56)	
Week 4	LS Mean (SEM)	-0.82 (0.507)	0.39 (0.789)	-1.21 (0.944)	0.2145
	95% CI for LS MEAN	(-1.89, 0.24)	(-1.27, 2.05)	(-3.20, 0.77)	
Week 8	LS Mean (SEM)	-0.13 (0.819)	-0.91 (1.312)	0.78 (1.545)	0.6217
	95% CI for LS MEAN	(-1.87, 1.61)	(-3.69, 1.88)	(-2.50, 4.05)	
Week 12	LS Mean (SEM)	-0.91 (0.737)	0.72 (1.062)	-1.63 (1.298)	0.2281
	95% CI for LS MEAN	(-2.48, 0.66)	(-1.55, 3.00)	(-4.40, 1.14)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.9.3
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by Age at Informed Consent
(< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.4452
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.9.4
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Europe

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.90 (0.440)	-0.73 (0.604)	-0.17 (0.752)	0.8245
	95% CI for LS MEAN	(-1.80, 0.00)	(-1.96, 0.51)	(-1.71, 1.37)	
Week 4	LS Mean (SEM)	-1.51 (0.458)	-0.63 (0.646)	-0.87 (0.797)	0.2810
	95% CI for LS MEAN	(-2.44, -0.57)	(-1.95, 0.69)	(-2.50, 0.75)	
Week 8	LS Mean (SEM)	-1.80 (0.631)	0.47 (0.957)	-2.27 (1.147)	0.0597
	95% CI for LS MEAN	(-3.11, -0.49)	(-1.51, 2.45)	(-4.64, 0.10)	
Week 12	LS Mean (SEM)	-1.95 (0.556)	-0.80 (0.747)	-1.15 (0.933)	0.2273
	95% CI for LS MEAN	(-3.08, -0.82)	(-2.32, 0.72)	(-3.05, 0.75)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.9.4
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.91 (0.380)	0.13 (0.620)	-1.04 (0.706)	0.1497
	95% CI for LS MEAN	(-1.68, -0.14)	(-1.12, 1.38)	(-2.47, 0.39)	
Week 4	LS Mean (SEM)	-1.24 (0.407)	-0.57 (0.644)	-0.67 (0.747)	0.3752
	95% CI for LS MEAN	(-2.06, -0.42)	(-1.87, 0.73)	(-2.18, 0.84)	
Week 8	LS Mean (SEM)	-1.63 (0.450)	-0.54 (0.694)	-1.09 (0.813)	0.1893
	95% CI for LS MEAN	(-2.54, -0.72)	(-1.95, 0.87)	(-2.74, 0.56)	
Week 12	LS Mean (SEM)	-1.60 (0.455)	-0.15 (0.701)	-1.45 (0.823)	0.0860
	95% CI for LS MEAN	(-2.52, -0.68)	(-1.57, 1.27)	(-3.11, 0.21)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.9.4
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.7314
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.9.5
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of
Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.24 (0.546)	-0.50 (0.837)	-0.74 (0.971)	0.4522
	95% CI for LS MEAN	(-2.35, -0.13)	(-2.20, 1.20)	(-2.72, 1.24)	
Week 4	LS Mean (SEM)	-2.21 (0.514)	-0.98 (0.757)	-1.23 (0.890)	0.1776
	95% CI for LS MEAN	(-3.26, -1.16)	(-2.53, 0.57)	(-3.05, 0.59)	
Week 8	LS Mean (SEM)	-1.94 (0.556)	0.48 (0.847)	-2.42 (0.991)	0.0204
	95% CI for LS MEAN	(-3.07, -0.81)	(-1.25, 2.21)	(-4.44, -0.40)	
Week 12	LS Mean (SEM)	-2.48 (0.504)	-0.48 (0.723)	-2.00 (0.857)	0.0267
	95% CI for LS MEAN	(-3.51, -1.45)	(-1.96, 1.00)	(-3.75, -0.25)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.9.5

Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 31)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.70 (0.284)	0.02 (0.418)	-0.72 (0.505)	0.1638
	95% CI for LS MEAN	(-1.27, -0.12)	(-0.83, 0.87)	(-1.74, 0.31)	
Week 4	LS Mean (SEM)	-0.72 (0.369)	-0.20 (0.550)	-0.52 (0.663)	0.4363
	95% CI for LS MEAN	(-1.46, 0.03)	(-1.31, 0.91)	(-1.86, 0.82)	
Week 8	LS Mean (SEM)	-1.40 (0.457)	-0.59 (0.676)	-0.81 (0.816)	0.3284
	95% CI for LS MEAN	(-2.33, -0.47)	(-1.96, 0.78)	(-2.46, 0.85)	
Week 12	LS Mean (SEM)	-1.18 (0.494)	-0.34 (0.707)	-0.84 (0.863)	0.3341
	95% CI for LS MEAN	(-2.18, -0.19)	(-1.77, 1.09)	(-2.59, 0.90)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.9.5

Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.4749
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.9.6
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of
Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.44 (0.311)	-0.18 (0.465)	-0.26 (0.563)	0.6485
	95% CI for LS MEAN	(-1.07, 0.19)	(-1.12, 0.76)	(-1.39, 0.88)	
Week 4	LS Mean (SEM)	-1.17 (0.352)	-0.72 (0.525)	-0.45 (0.636)	0.4789
	95% CI for LS MEAN	(-1.88, -0.46)	(-1.78, 0.34)	(-1.73, 0.83)	
Week 8	LS Mean (SEM)	-0.85 (0.465)	-0.39 (0.718)	-0.46 (0.858)	0.5957
	95% CI for LS MEAN	(-1.79, 0.09)	(-1.84, 1.05)	(-2.19, 1.27)	
Week 12	LS Mean (SEM)	-1.50 (0.429)	-0.05 (0.610)	-1.45 (0.749)	0.0595
	95% CI for LS MEAN	(-2.36, -0.64)	(-1.28, 1.18)	(-2.96, 0.06)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.9.6
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of
Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.34 (0.489)	-0.22 (0.746)	-1.12 (0.864)	0.2078
	95% CI for LS MEAN	(-2.34, -0.33)	(-1.75, 1.31)	(-2.89, 0.66)	
Week 4	LS Mean (SEM)	-1.34 (0.531)	-0.16 (0.797)	-1.18 (0.935)	0.2181
	95% CI for LS MEAN	(-2.43, -0.26)	(-1.79, 1.47)	(-3.10, 0.74)	
Week 8	LS Mean (SEM)	-2.52 (0.514)	0.21 (0.745)	-2.73 (0.879)	0.0058
	95% CI for LS MEAN	(-3.59, -1.45)	(-1.35, 1.77)	(-4.58, -0.89)	
Week 12	LS Mean (SEM)	-1.87 (0.600)	-0.94 (0.884)	-0.93 (1.049)	0.3824
	95% CI for LS MEAN	(-3.10, -0.65)	(-2.75, 0.87)	(-3.08, 1.22)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.9.6
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of
Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.5241
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.9.7
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by Background C5 Inhibitor Therapy
(Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 20)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.70 (0.455)	-0.52 (0.712)	-0.18 (0.837)	0.8280
	95% CI for LS MEAN	(-1.64, 0.24)	(-1.98, 0.94)	(-1.91, 1.54)	
Week 4	LS Mean (SEM)	-1.23 (0.421)	-0.31 (0.611)	-0.93 (0.740)	0.2215
	95% CI for LS MEAN	(-2.10, -0.37)	(-1.56, 0.95)	(-2.45, 0.59)	
Week 8	LS Mean (SEM)	-0.76 (0.727)	0.16 (1.115)	-0.92 (1.329)	0.4977
	95% CI for LS MEAN	(-2.26, 0.75)	(-2.14, 2.46)	(-3.66, 1.83)	
Week 12	LS Mean (SEM)	-1.38 (0.607)	-0.71 (0.813)	-0.67 (1.012)	0.5139
	95% CI for LS MEAN	(-2.62, -0.13)	(-2.37, 0.96)	(-2.75, 1.41)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.9.7
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by Background C5 Inhibitor Therapy
(Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.08 (0.354)	-0.11 (0.534)	-0.97 (0.633)	0.1326
	95% CI for LS MEAN	(-1.80, -0.37)	(-1.19, 0.96)	(-2.25, 0.31)	
Week 4	LS Mean (SEM)	-1.46 (0.408)	-0.76 (0.640)	-0.70 (0.751)	0.3544
	95% CI for LS MEAN	(-2.28, -0.64)	(-2.04, 0.53)	(-2.22, 0.81)	
Week 8	LS Mean (SEM)	-1.93 (0.423)	-0.13 (0.646)	-1.80 (0.765)	0.0240
	95% CI for LS MEAN	(-2.78, -1.07)	(-1.44, 1.18)	(-3.35, -0.25)	
Week 12	LS Mean (SEM)	-2.03 (0.421)	-0.26 (0.649)	-1.77 (0.766)	0.0256
	95% CI for LS MEAN	(-2.87, -1.18)	(-1.56, 1.05)	(-3.31, -0.23)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.9.7
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by Background C5 Inhibitor Therapy
(Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.5302
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

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For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.9.8

Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.61 (1.323)	-0.30 (1.062)	-0.31 (1.995)	0.8784
	95% CI for LS MEAN	(-3.55, 2.33)	(-2.67, 2.08)	(-4.72, 4.09)	
Week 4	LS Mean (SEM)	-1.23 (1.299)	0.45 (1.000)	-1.69 (1.947)	0.4056
	95% CI for LS MEAN	(-4.14, 1.67)	(-1.75, 2.66)	(-6.01, 2.63)	
Week 8	LS Mean (SEM)	-1.48 (1.413)	-0.80 (1.272)	-0.69 (2.172)	0.7563
	95% CI for LS MEAN	(-4.54, 1.57)	(-3.58, 1.98)	(-5.36, 3.98)	
Week 12	LS Mean (SEM)	-1.86 (1.579)	1.20 (1.616)	-3.06 (2.491)	0.2391
	95% CI for LS MEAN	(-5.23, 1.51)	(-2.36, 4.77)	(-8.40, 2.28)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.9.8
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 47)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.97 (0.306)	-0.12 (0.477)	-0.85 (0.564)	0.1362
	95% CI for LS MEAN	(-1.59, -0.36)	(-1.08, 0.83)	(-1.98, 0.28)	
Week 4	LS Mean (SEM)	-1.38 (0.342)	-0.66 (0.528)	-0.72 (0.626)	0.2564
	95% CI for LS MEAN	(-2.06, -0.70)	(-1.72, 0.39)	(-1.97, 0.53)	
Week 8	LS Mean (SEM)	-1.59 (0.390)	0.16 (0.608)	-1.75 (0.719)	0.0184
	95% CI for LS MEAN	(-2.37, -0.81)	(-1.06, 1.38)	(-3.19, -0.31)	
Week 12	LS Mean (SEM)	-1.70 (0.361)	-0.66 (0.539)	-1.04 (0.646)	0.1116
	95% CI for LS MEAN	(-2.42, -0.98)	(-1.74, 0.41)	(-2.33, 0.25)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.9.8

Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.8868
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:54:14

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Table GERMANY.2.5.9.9
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.45 (0.338)	0.17 (0.461)	-0.62 (0.536)	0.2557
	95% CI for LS MEAN	(-1.14, 0.24)	(-0.77, 1.12)	(-1.72, 0.48)	
Week 4	LS Mean (SEM)	-1.20 (0.440)	-0.73 (0.631)	-0.48 (0.744)	0.5272
	95% CI for LS MEAN	(-2.10, -0.30)	(-2.02, 0.56)	(-2.00, 1.05)	
Week 8	LS Mean (SEM)	-1.37 (0.511)	-0.43 (0.698)	-0.94 (0.844)	0.2750
	95% CI for LS MEAN	(-2.41, -0.32)	(-1.86, 1.01)	(-2.67, 0.79)	
Week 12	LS Mean (SEM)	-1.40 (0.555)	0.27 (0.775)	-1.68 (0.934)	0.0829
	95% CI for LS MEAN	(-2.53, -0.27)	(-1.31, 1.86)	(-3.59, 0.23)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:54:15

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.9.9
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

White

Visit	Statistics	Danicopan (N = 26)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.29 (0.495)	-0.75 (0.800)	-0.55 (0.942)	0.5653
	95% CI for LS MEAN	(-2.30, -0.28)	(-2.38, 0.88)	(-2.47, 1.37)	
Week 4	LS Mean (SEM)	-1.90 (0.441)	-0.60 (0.717)	-1.31 (0.843)	0.1318
	95% CI for LS MEAN	(-2.81, -1.00)	(-2.06, 0.87)	(-3.03, 0.42)	
Week 8	LS Mean (SEM)	-1.53 (0.575)	0.27 (0.949)	-1.80 (1.111)	0.1165
	95% CI for LS MEAN	(-2.72, -0.34)	(-1.68, 2.22)	(-4.09, 0.48)	
Week 12	LS Mean (SEM)	-1.93 (0.510)	-1.10 (0.799)	-0.83 (0.949)	0.3875
	95% CI for LS MEAN	(-2.97, -0.89)	(-2.73, 0.53)	(-2.76, 1.10)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:54:15

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.9.9
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	0.11 (0.811)	-0.82 (1.206)	0.93 (1.506)	0.5410
	95% CI for LS MEAN	(-1.56, 1.78)	(-3.30, 1.66)	(-2.16, 4.03)	
Week 4	LS Mean (SEM)	1.25 (0.811)	-0.24 (1.020)	1.49 (1.318)	0.2675
	95% CI for LS MEAN	(-0.41, 2.92)	(-2.35, 1.87)	(-1.22, 4.21)	
Week 8	LS Mean (SEM)	-0.99 (1.030)	1.46 (2.321)	-2.45 (2.605)	0.3585
	95% CI for LS MEAN	(-3.11, 1.13)	(-3.36, 6.27)	(-7.86, 2.97)	
Week 12	LS Mean (SEM)	-0.89 (0.811)	-0.74 (1.020)	-0.15 (1.318)	0.9111
	95% CI for LS MEAN	(-2.56, 0.78)	(-2.85, 1.37)	(-2.86, 2.56)	

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:54:15

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.9.9
Change from Baseline in WPAI:ANS During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Subgroup by treatment interaction p-value	0.9990
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline WPAI:ANS value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

WPAI:ANS is Work Productivity and Activity Impairment Questionnaire: Anemic Symptoms V2.0. All the Questionnaires are collected in past 7 days. Only how much anemic symptoms affect ability on regular daily activities, other than work was reported.

For how much anemic symptoms affect ability on regular daily activities, other than work, the data were collected as 10 level from 0 which indicate Anemic symptoms had no effect on daily activities to 10 which dicate Anemic symptoms completely prevented patients from doing daily activites.

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:54:15

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

2 Gesundheitsbezogene Lebensqualität

2.1 Gesundheitsbezogene Lebensqualität anhand des EORTC QLQ-C30

2.1.1 Rücklaufquoten des EORTC QLQ-C30

Die Rücklaufquoten sind unter Abschnitt 1.6.1 Rücklaufquoten des EORTC QLQ-C30 dargestellt

Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

2 Gesundheitsbezogene Lebensqualität

2.1 Gesundheitsbezogene Lebensqualität anhand des EORTC QLQ-C30

2.1.2 Veränderung des EORTC QLQ-C30 Gesundheitsstatus und der Funktionskalen im Vergleich zu Baseline

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Table GERMANY.2.5.11.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Global Health Status/QoL

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	57		24		81	
Mean (SD)	58.33 (20.168)		55.21 (17.517)		57.41 (19.365)	
Median	58.33		54.17		58.33	
Min, Max	16.7, 100.0		16.7, 91.7		16.7, 100.0	
Week 2						
n	57	57	25	23		
Mean (SD)	63.89 (19.245)	5.56 (17.490)	58.33 (15.775)	3.26 (18.759)		
Median	66.67	8.33	58.33	0.00		
Min, Max	16.7, 100.0	-50.0, 41.7	25.0, 83.3	-25.0, 50.0		
Week 4						
n	57	57	25	24		
Mean (SD)	66.81 (19.511)	8.48 (16.554)	56.00 (21.452)	-0.35 (16.572)		
Median	66.67	0.00	50.00	0.00		
Min, Max	25.0, 100.0	-16.7, 50.0	25.0, 100.0	-41.7, 41.7		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.11.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Global Health Status/QoL

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	45	45	19	18		
Mean (SD)	67.22 (21.933)	8.89 (22.571)	58.77 (22.648)	1.85 (21.685)		
Median	66.67	8.33	66.67	0.00		
Min, Max	16.7, 100.0	-58.3, 50.0	16.7, 83.3	-33.3, 41.7		
Week 12						
n	52	52	25	24		
Mean (SD)	69.87 (18.827)	12.18 (15.521)	62.33 (17.031)	6.60 (18.386)		
Median	66.67	12.50	66.67	4.17		
Min, Max	33.3, 100.0	-16.7, 41.7	33.3, 91.7	-25.0, 41.7		
Week 24						
n	49	49	24	23		
Mean (SD)	68.88 (19.378)	10.03 (17.262)	66.32 (18.952)	10.51 (15.121)		
Median	66.67	16.67	66.67	8.33		
Min, Max	16.7, 100.0	-33.3, 41.7	16.7, 100.0	-16.7, 41.7		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:48:23

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Table GERMANY.2.5.11.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Global Health Status/QoL

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	46	46	21	21	67	67
Mean (SD)	68.66 (19.578)	10.14 (16.093)	62.70 (23.217)	6.35 (18.048)	66.79 (20.795)	8.96 (16.686)
Median	66.67	8.33	66.67	8.33	66.67	8.33
Min, Max	33.3, 100.0	-41.7, 33.3	16.7, 100.0	-33.3, 33.3	16.7, 100.0	-41.7, 33.3
Week 56						
n	34	34	17	16	51	50
Mean (SD)	66.91 (18.632)	7.35 (16.253)	61.27 (20.612)	1.04 (20.833)	65.03 (19.294)	5.33 (17.878)
Median	66.67	0.00	66.67	0.00	66.67	0.00
Min, Max	33.3, 100.0	-16.7, 41.7	8.3, 83.3	-25.0, 41.7	8.3, 100.0	-25.0, 41.7
Week 72						
n	20	20	12	11	32	31
Mean (SD)	69.58 (15.829)	7.92 (22.697)	62.50 (22.891)	2.27 (25.025)	66.93 (18.752)	5.91 (23.293)
Median	66.67	4.17	66.67	0.00	66.67	0.00
Min, Max	33.3, 100.0	-50.0, 41.7	16.7, 91.7	-33.3, 50.0	16.7, 100.0	-50.0, 50.0

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.11.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Global Health Status/QoL

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	74.40 (15.143)	8.33 (15.676)	61.46 (22.244)	4.17 (23.987)	69.70 (18.642)	6.82 (18.658)
Median	75.00	12.50	66.67	4.17	66.67	8.33
Min, Max	41.7, 100.0	-16.7, 33.3	16.7, 83.3	-33.3, 41.7	16.7, 100.0	-33.3, 41.7
Week 104						
n	4	4	2	2	6	6
Mean (SD)	81.25 (21.916)	8.33 (16.667)	79.17 (5.893)	25.00 (23.570)	80.56 (17.213)	13.89 (18.758)
Median	83.33	0.00	79.17	25.00	79.17	4.17
Min, Max	58.3, 100.0	0.0, 33.3	75.0, 83.3	8.3, 41.7	58.3, 100.0	0.0, 41.7

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.11.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Physical Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	57		24		81	
Mean (SD)	73.33 (18.814)		65.28 (20.523)		70.95 (19.559)	
Median	80.00		66.67		73.33	
Min, Max	33.3, 100.0		26.7, 100.0		26.7, 100.0	
Week 2						
n	56	56	25	23		
Mean (SD)	78.93 (17.651)	5.95 (12.039)	66.67 (18.359)	1.16 (12.002)		
Median	83.33	0.00	66.67	0.00		
Min, Max	26.7, 100.0	-20.0, 46.7	26.7, 100.0	-13.3, 40.0		
Week 4						
n	57	57	25	24		
Mean (SD)	84.21 (15.360)	10.88 (14.619)	66.93 (21.036)	0.83 (17.397)		
Median	86.67	6.67	66.67	0.00		
Min, Max	40.0, 100.0	-20.0, 60.0	26.7, 100.0	-26.7, 53.3		

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Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.11.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Physical Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	45	45	19	18		
Mean (SD)	83.56 (15.609)	9.78 (14.744)	70.53 (20.884)	4.44 (17.112)		
Median	86.67	13.33	73.33	0.00		
Min, Max	33.3, 100.0	-26.7, 46.7	20.0, 100.0	-26.7, 46.7		
Week 12						
n	52	52	25	24		
Mean (SD)	85.13 (15.260)	11.15 (13.574)	66.13 (20.450)	0.28 (17.636)		
Median	86.67	6.67	73.33	0.00		
Min, Max	26.7, 100.0	-6.7, 46.7	20.0, 100.0	-33.3, 46.7		
Week 24						
n	49	49	24	23		
Mean (SD)	85.85 (14.822)	11.29 (16.789)	77.78 (22.125)	10.43 (13.154)		
Median	86.67	13.33	86.67	13.33		
Min, Max	40.0, 100.0	-33.3, 60.0	26.7, 100.0	-6.7, 40.0		

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Modified Randomized Set

Physical Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	46	46	21	21	67	67
Mean (SD)	83.77 (17.535)	9.28 (15.168)	73.65 (24.808)	4.76 (14.475)	80.60 (20.457)	7.86 (14.994)
Median	86.67	6.67	73.33	6.67	86.67	6.67
Min, Max	13.3, 100.0	-26.7, 53.3	20.0, 100.0	-26.7, 26.7	13.3, 100.0	-26.7, 53.3
Week 56						
n	34	34	17	16	51	50
Mean (SD)	83.33 (16.268)	9.22 (14.863)	79.22 (17.302)	6.25 (14.701)	81.96 (16.562)	8.27 (14.727)
Median	86.67	10.00	80.00	6.67	86.67	10.00
Min, Max	46.7, 100.0	-20.0, 60.0	46.7, 100.0	-20.0, 33.3	46.7, 100.0	-20.0, 60.0
Week 72						
n	21	21	12	11	33	32
Mean (SD)	84.44 (13.878)	10.16 (16.683)	75.00 (25.126)	6.06 (18.001)	81.01 (18.939)	8.75 (16.970)
Median	86.67	13.33	80.00	13.33	86.67	13.33
Min, Max	53.3, 100.0	-13.3, 60.0	6.7, 100.0	-40.0, 20.0	6.7, 100.0	-40.0, 60.0

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Table GERMANY.2.5.11.1
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During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Physical Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	88.10 (12.858)	10.95 (10.972)	74.17 (24.543)	7.50 (17.252)	83.03 (18.713)	9.70 (13.290)
Median	90.00	6.67	76.67	3.33	86.67	6.67
Min, Max	60.0, 100.0	-6.7, 33.3	33.3, 100.0	-13.3, 33.3	33.3, 100.0	-13.3, 33.3
Week 104						
n	4	4	2	2	6	6
Mean (SD)	91.67 (12.620)	8.33 (6.383)	90.00 (4.714)	20.00 (18.856)	91.11 (10.037)	12.22 (11.483)
Median	96.67	10.00	90.00	20.00	93.33	10.00
Min, Max	73.3, 100.0	0.0, 13.3	86.7, 93.3	6.7, 33.3	73.3, 100.0	0.0, 33.3

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Table GERMANY.2.5.11.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Role Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	57		24		81	
Mean (SD)	67.25 (27.452)		63.19 (28.224)		66.05 (27.569)	
Median	66.67		66.67		66.67	
Min, Max	0.0, 100.0		16.7, 100.0		0.0, 100.0	
Week 2						
n	55	55	24	22		
Mean (SD)	75.15 (28.305)	8.48 (23.542)	68.06 (27.766)	2.27 (17.286)		
Median	83.33	0.00	66.67	0.00		
Min, Max	0.0, 100.0	-50.0, 66.7	0.0, 100.0	-33.3, 33.3		
Week 4						
n	57	57	24	23		
Mean (SD)	81.29 (24.002)	14.04 (27.420)	68.06 (26.429)	2.17 (25.773)		
Median	83.33	16.67	66.67	0.00		
Min, Max	0.0, 100.0	-66.7, 83.3	16.7, 100.0	-33.3, 83.3		

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Table GERMANY.2.5.11.1
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Modified Randomized Set

Role Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	45	45	18	17		
Mean (SD)	81.48 (20.787)	14.81 (23.630)	67.59 (26.490)	-2.94 (29.009)		
Median	83.33	16.67	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 83.3	16.7, 100.0	-66.7, 66.7		
Week 12						
n	52	52	24	23		
Mean (SD)	82.05 (22.348)	14.42 (24.260)	68.75 (28.789)	4.35 (34.163)		
Median	91.67	16.67	75.00	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 100.0	-66.7, 66.7		
Week 24						
n	49	49	24	23		
Mean (SD)	77.55 (28.576)	9.52 (29.659)	74.31 (29.887)	10.14 (37.179)		
Median	83.33	0.00	83.33	16.67		
Min, Max	0.0, 100.0	-66.7, 83.3	0.0, 100.0	-100.0, 66.7		

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Table GERMANY.2.5.11.1
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Modified Randomized Set

Role Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	46	46	21	21	67	67
Mean (SD)	80.07 (24.750)	10.14 (26.171)	68.25 (34.522)	5.56 (25.999)	76.37 (28.449)	8.71 (26.008)
Median	91.67	0.00	66.67	0.00	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-50.0, 50.0	0.0, 100.0	-66.7, 66.7
Week 56						
n	34	34	17	16	51	50
Mean (SD)	74.51 (26.026)	3.92 (27.542)	81.37 (15.459)	16.67 (27.217)	76.80 (23.113)	8.00 (27.815)
Median	66.67	0.00	83.33	16.67	66.67	0.00
Min, Max	0.0, 100.0	-83.3, 66.7	66.7, 100.0	-33.3, 66.7	0.0, 100.0	-83.3, 66.7
Week 72						
n	21	21	11	10	32	31
Mean (SD)	80.95 (27.531)	15.08 (25.769)	77.27 (21.438)	20.00 (25.820)	79.69 (25.306)	16.67 (25.459)
Median	100.00	16.67	83.33	25.00	91.67	16.67
Min, Max	0.0, 100.0	-33.3, 66.7	50.0, 100.0	-16.7, 66.7	0.0, 100.0	-33.3, 66.7

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Table GERMANY.2.5.11.1
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Modified Randomized Set

Role Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	85.71 (22.510)	13.10 (20.856)	70.83 (27.817)	12.50 (29.209)	80.30 (25.006)	12.88 (23.532)
Median	100.00	0.00	75.00	0.00	91.67	0.00
Min, Max	33.3, 100.0	-16.7, 50.0	16.7, 100.0	-16.7, 66.7	16.7, 100.0	-16.7, 66.7
Week 104						
n	4	4	2	2	6	6
Mean (SD)	87.50 (25.000)	4.17 (8.333)	83.33 (23.570)	33.33 (23.570)	86.11 (22.153)	13.89 (19.484)
Median	100.00	0.00	83.33	33.33	100.00	8.33
Min, Max	50.0, 100.0	0.0, 16.7	66.7, 100.0	16.7, 50.0	50.0, 100.0	0.0, 50.0

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Table GERMANY.2.5.11.1
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Modified Randomized Set

Emotional Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	57		24		81	
Mean (SD)	79.68 (22.384)		73.96 (24.365)		77.98 (22.985)	
Median	83.33		83.33		83.33	
Min, Max	0.0, 100.0		8.3, 100.0		0.0, 100.0	
Week 2						
n	56	56	25	23		
Mean (SD)	84.38 (18.192)	4.76 (15.558)	74.00 (21.153)	1.09 (15.139)		
Median	91.67	0.00	75.00	0.00		
Min, Max	33.3, 100.0	-25.0, 58.3	33.3, 100.0	-33.3, 25.0		
Week 4						
n	57	57	25	24		
Mean (SD)	84.06 (22.398)	4.39 (18.905)	77.33 (22.116)	2.43 (16.753)		
Median	91.67	0.00	83.33	0.00		
Min, Max	0.0, 100.0	-66.7, 58.3	25.0, 100.0	-33.3, 33.3		

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Table GERMANY.2.5.11.1
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Modified Randomized Set

Emotional Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	45	45	19	18		
Mean (SD)	85.19 (16.938)	6.67 (17.462)	83.33 (20.601)	5.56 (13.098)		
Median	91.67	0.00	83.33	4.17		
Min, Max	41.7, 100.0	-25.0, 58.3	16.7, 100.0	-16.7, 33.3		
Week 12						
n	52	52	25	24		
Mean (SD)	87.34 (18.560)	7.53 (14.125)	74.33 (23.805)	0.35 (18.138)		
Median	100.00	4.17	75.00	0.00		
Min, Max	16.7, 100.0	-16.7, 66.7	0.0, 100.0	-50.0, 33.3		
Week 24						
n	49	49	24	23		
Mean (SD)	84.86 (22.352)	5.44 (18.677)	80.90 (17.971)	6.88 (18.575)		
Median	91.67	0.00	83.33	8.33		
Min, Max	0.0, 100.0	-66.7, 66.7	33.3, 100.0	-33.3, 50.0		

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Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.11.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Emotional Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	46	46	21	21	67	67
Mean (SD)	85.33 (21.744)	4.71 (18.975)	77.78 (18.696)	4.37 (21.509)	82.96 (20.993)	4.60 (19.639)
Median	91.67	4.17	75.00	0.00	91.67	0.00
Min, Max	0.0, 100.0	-66.7, 58.3	25.0, 100.0	-25.0, 66.7	0.0, 100.0	-66.7, 66.7
Week 56						
n	34	34	17	16	51	50
Mean (SD)	82.35 (23.904)	3.68 (21.923)	78.92 (18.190)	8.85 (25.904)	81.21 (22.038)	5.33 (23.131)
Median	91.67	0.00	83.33	0.00	91.67	0.00
Min, Max	0.0, 100.0	-66.7, 50.0	41.7, 100.0	-16.7, 75.0	0.0, 100.0	-66.7, 75.0
Week 72						
n	20	20	12	11	32	31
Mean (SD)	90.83 (13.218)	5.42 (16.058)	76.39 (24.317)	9.09 (17.262)	85.42 (19.167)	6.72 (16.304)
Median	100.00	0.00	75.00	8.33	91.67	8.33
Min, Max	58.3, 100.0	-33.3, 33.3	25.0, 100.0	-25.0, 33.3	25.0, 100.0	-33.3, 33.3

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Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.11.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Emotional Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	91.07 (12.431)	6.55 (10.928)	77.08 (24.701)	16.67 (35.355)	85.98 (18.614)	10.23 (22.703)
Median	95.83	4.17	87.50	20.83	91.67	8.33
Min, Max	66.7, 100.0	-8.3, 25.0	33.3, 100.0	-33.3, 83.3	33.3, 100.0	-33.3, 83.3
Week 104						
n	4	4	2	2	6	6
Mean (SD)	95.83 (8.333)	2.08 (4.167)	62.50 (41.248)	25.00 (82.496)	84.72 (26.043)	9.72 (38.879)
Median	100.00	0.00	62.50	25.00	95.83	0.00
Min, Max	83.3, 100.0	0.0, 8.3	33.3, 91.7	-33.3, 83.3	33.3, 100.0	-33.3, 83.3

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Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.11.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Cognitive Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	57		24		81	
Mean (SD)	78.65 (22.659)		70.14 (26.457)		76.13 (23.999)	
Median	83.33		66.67		83.33	
Min, Max	16.7, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	56	56	24	23		
Mean (SD)	83.04 (18.116)	4.46 (16.964)	73.61 (27.766)	4.35 (24.214)		
Median	83.33	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-16.7, 66.7	0.0, 100.0	-33.3, 66.7		
Week 4						
n	57	57	25	24		
Mean (SD)	84.50 (17.778)	5.85 (18.498)	71.33 (29.861)	0.69 (24.808)		
Median	83.33	0.00	83.33	0.00		
Min, Max	16.7, 100.0	-50.0, 50.0	0.0, 100.0	-33.3, 50.0		

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Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.11.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Cognitive Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	45	45	19	18		
Mean (SD)	84.07 (16.266)	7.78 (17.979)	77.19 (26.765)	9.26 (20.787)		
Median	83.33	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 50.0		
Week 12						
n	52	52	25	24		
Mean (SD)	84.94 (17.545)	7.05 (15.249)	75.33 (25.963)	4.86 (22.778)		
Median	83.33	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-16.7, 50.0	16.7, 100.0	-33.3, 50.0		
Week 24						
n	49	49	24	23		
Mean (SD)	82.31 (24.154)	4.76 (20.694)	78.47 (22.241)	7.25 (17.997)		
Median	83.33	0.00	83.33	0.00		
Min, Max	0.0, 100.0	-66.7, 50.0	33.3, 100.0	-33.3, 33.3		

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Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:48:23

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Table GERMANY.2.5.11.1
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During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Cognitive Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	46	46	21	21	67	67
Mean (SD)	82.25 (22.334)	5.07 (20.449)	70.63 (27.338)	-2.38 (19.211)	78.61 (24.413)	2.74 (20.225)
Median	83.33	0.00	66.67	0.00	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 50.0	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 50.0
Week 56						
n	34	34	17	16	51	50
Mean (SD)	84.31 (22.073)	4.41 (19.380)	73.53 (31.213)	1.04 (21.490)	80.72 (25.685)	3.33 (19.920)
Median	100.00	0.00	83.33	0.00	83.33	0.00
Min, Max	16.7, 100.0	-50.0, 50.0	0.0, 100.0	-50.0, 33.3	0.0, 100.0	-50.0, 50.0
Week 72						
n	20	20	11	10	31	30
Mean (SD)	91.67 (12.681)	6.67 (15.672)	74.24 (31.059)	6.67 (16.102)	85.48 (22.254)	6.67 (15.536)
Median	100.00	0.00	83.33	8.33	100.00	0.00
Min, Max	66.7, 100.0	-16.7, 50.0	16.7, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 50.0

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Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.11.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Cognitive Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	94.05 (10.555)	7.14 (12.599)	60.42 (38.768)	0.00 (26.726)	81.82 (29.054)	4.55 (18.674)
Median	100.00	0.00	75.00	0.00	100.00	0.00
Min, Max	66.7, 100.0	0.0, 33.3	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 33.3
Week 104						
n	4	4	2	2	6	6
Mean (SD)	91.67 (16.667)	0.00 (0.000)	58.33 (35.355)	-8.33 (35.355)	80.56 (26.701)	-2.78 (16.387)
Median	100.00	0.00	58.33	-8.33	91.67	0.00
Min, Max	66.7, 100.0	0.0, 0.0	33.3, 83.3	-33.3, 16.7	33.3, 100.0	-33.3, 16.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Social Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	57		24		81	
Mean (SD)	75.44 (25.802)		69.44 (31.341)		73.66 (27.495)	
Median	83.33		83.33		83.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	57	57	24	22		
Mean (SD)	82.46 (20.992)	7.02 (22.260)	71.53 (28.437)	2.27 (19.447)		
Median	100.00	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-50.0, 83.3	0.0, 100.0	-33.3, 50.0		
Week 4						
n	57	57	24	23		
Mean (SD)	83.33 (20.893)	7.89 (21.154)	72.22 (27.217)	2.90 (22.837)		
Median	100.00	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 83.3	0.0, 100.0	-50.0, 50.0		

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Table GERMANY.2.5.11.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Social Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 8						
n	45	45	18	17		
Mean (SD)	86.30 (19.230)	10.00 (22.585)	80.56 (22.324)	5.88 (17.620)		
Median	100.00	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-50.0, 83.3	16.7, 100.0	-16.7, 33.3		
Week 12						
n	52	52	25	24		
Mean (SD)	86.54 (18.971)	10.58 (20.619)	68.00 (31.885)	-2.78 (26.314)		
Median	100.00	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 83.3	0.0, 100.0	-83.3, 33.3		
Week 24						
n	49	49	23	22		
Mean (SD)	81.97 (25.422)	5.78 (26.032)	75.36 (25.559)	7.58 (23.975)		
Median	100.00	0.00	83.33	8.33		
Min, Max	0.0, 100.0	-66.7, 83.3	0.0, 100.0	-33.3, 50.0		

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.11.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Social Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	46	46	21	21	67	67
Mean (SD)	80.07 (24.750)	2.54 (27.209)	61.11 (35.875)	-7.14 (45.207)	74.13 (29.770)	-0.50 (33.831)
Median	91.67	0.00	66.67	0.00	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 83.3	0.0, 100.0	-100.0, 50.0	0.0, 100.0	-100.0, 83.3
Week 56						
n	34	34	17	16	51	50
Mean (SD)	83.82 (21.511)	7.84 (25.037)	77.45 (19.491)	8.33 (28.545)	81.70 (20.885)	8.00 (25.916)
Median	100.00	0.00	83.33	0.00	83.33	0.00
Min, Max	33.3, 100.0	-50.0, 83.3	33.3, 100.0	-50.0, 66.7	33.3, 100.0	-50.0, 83.3
Week 72						
n	20	20	11	10	31	30
Mean (SD)	88.33 (19.571)	16.67 (25.363)	80.30 (25.624)	1.67 (32.820)	85.48 (21.834)	11.67 (28.416)
Median	100.00	8.33	83.33	16.67	100.00	16.67
Min, Max	33.3, 100.0	-16.7, 83.3	16.7, 100.0	-83.3, 33.3	16.7, 100.0	-83.3, 83.3

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:23

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.11.1
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Social Functioning

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	14	14	8	8	22	22
Mean (SD)	86.90 (16.249)	11.90 (23.956)	79.17 (17.252)	10.42 (33.259)	84.09 (16.649)	11.36 (26.917)
Median	100.00	0.00	66.67	16.67	91.67	8.33
Min, Max	66.7, 100.0	-16.7, 83.3	66.7, 100.0	-33.3, 66.7	66.7, 100.0	-33.3, 83.3
Week 104						
n	4	4	2	2	6	6
Mean (SD)	91.67 (16.667)	0.00 (27.217)	83.33 (23.570)	58.33 (11.785)	88.89 (17.213)	19.44 (37.143)
Median	100.00	0.00	83.33	58.33	100.00	16.67
Min, Max	66.7, 100.0	-33.3, 33.3	66.7, 100.0	50.0, 66.7	66.7, 100.0	-33.3, 66.7

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Source: ADaM.ADSL, ADaM.ADQS

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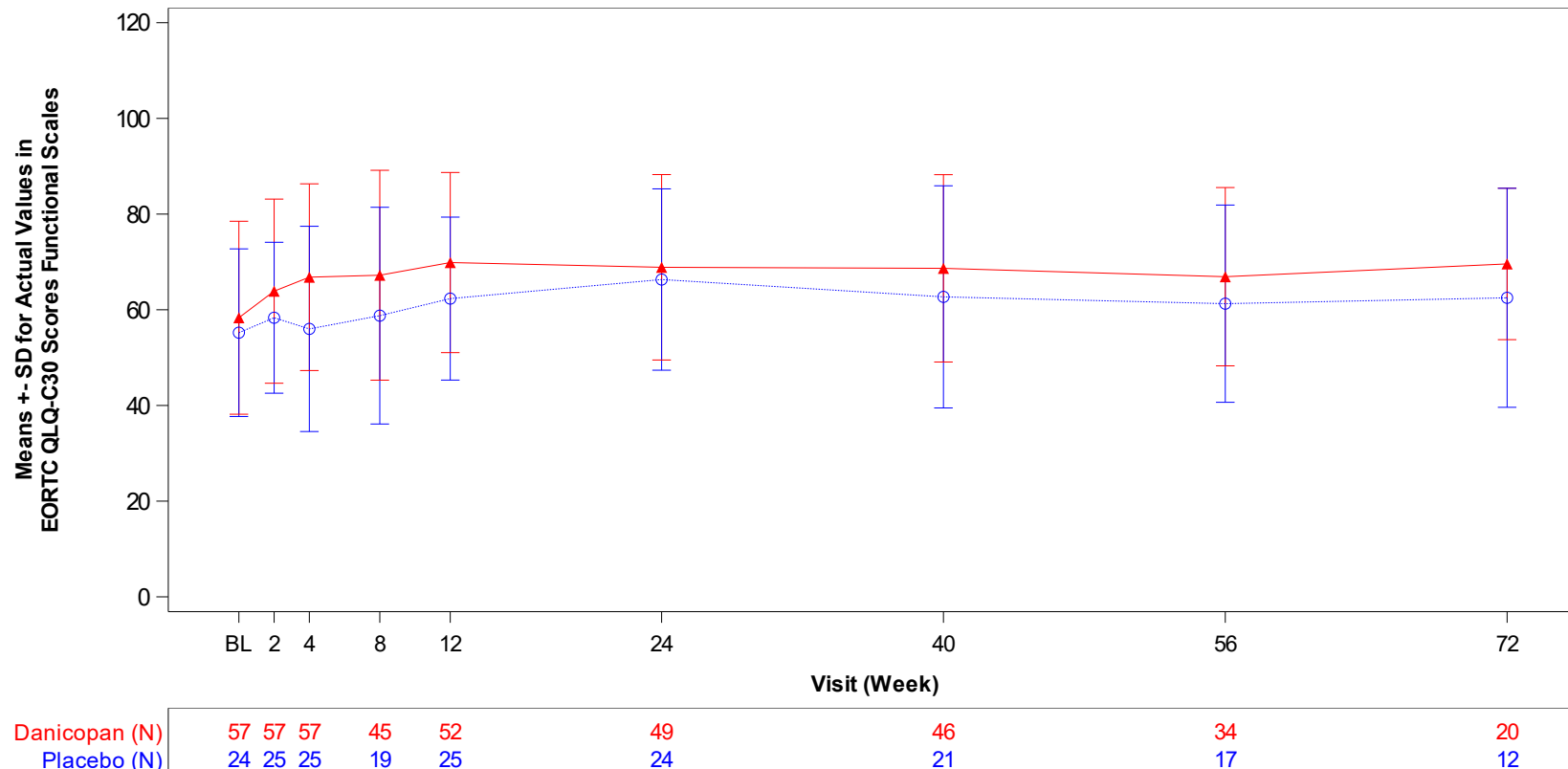
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Figure GERMANY.2.2.7.3
Plots of Mean \pm SD for Actual Values in EORTC QLQ-C30 Scores Functional Scales During 72 Weeks
Modified Randomized Set

Global Health Status/QoL



Baseline is defined as the last non-missing value prior to the first dose of study drug.
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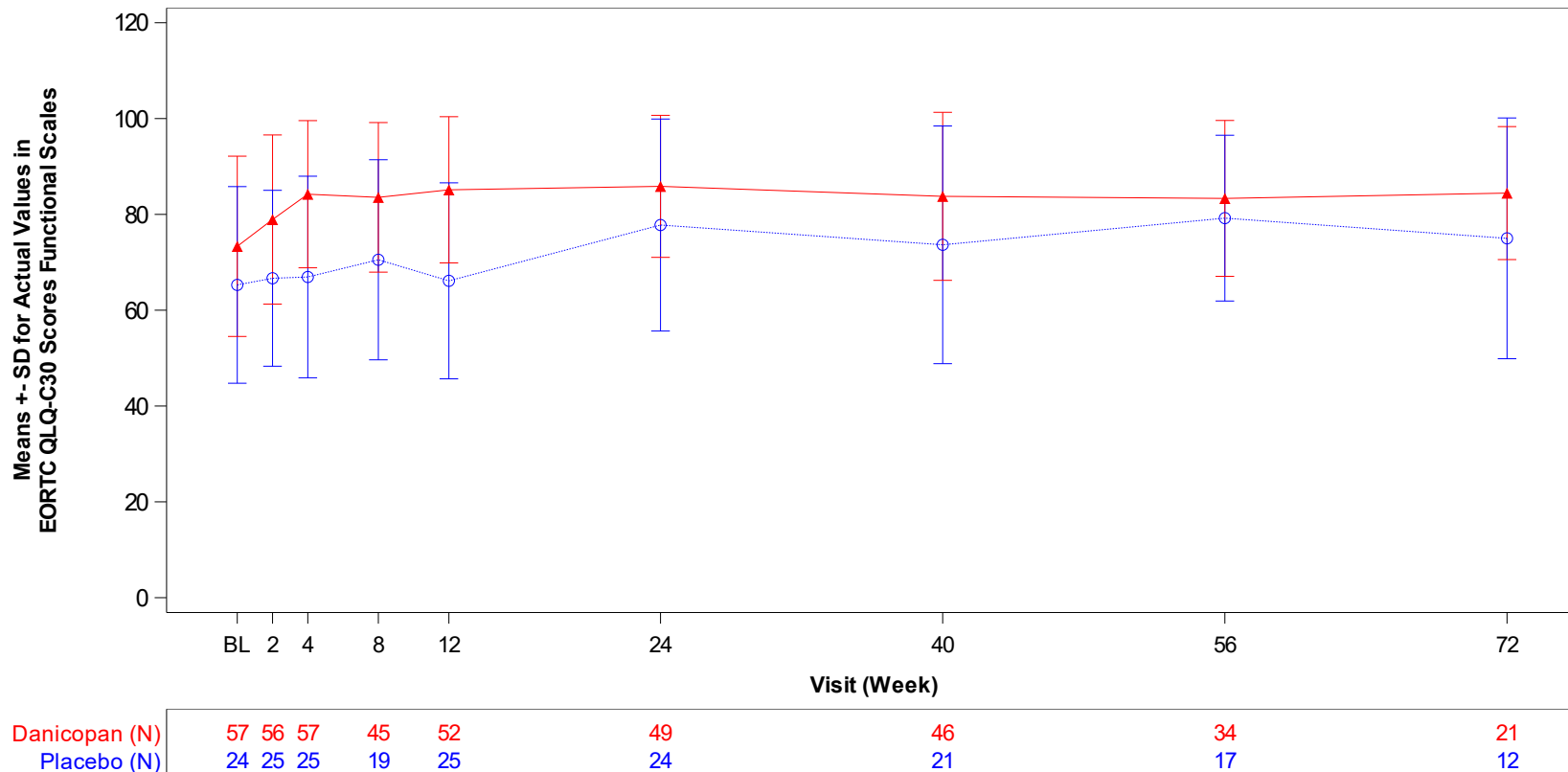
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Figure GERMANY.2.2.7.3
Plots of Mean \pm SD for Actual Values in EORTC QLQ-C30 Scores Functional Scales During 72 Weeks
Modified Randomized Set

Physical Functioning



Baseline is defined as the last non-missing value prior to the first dose of study drug.

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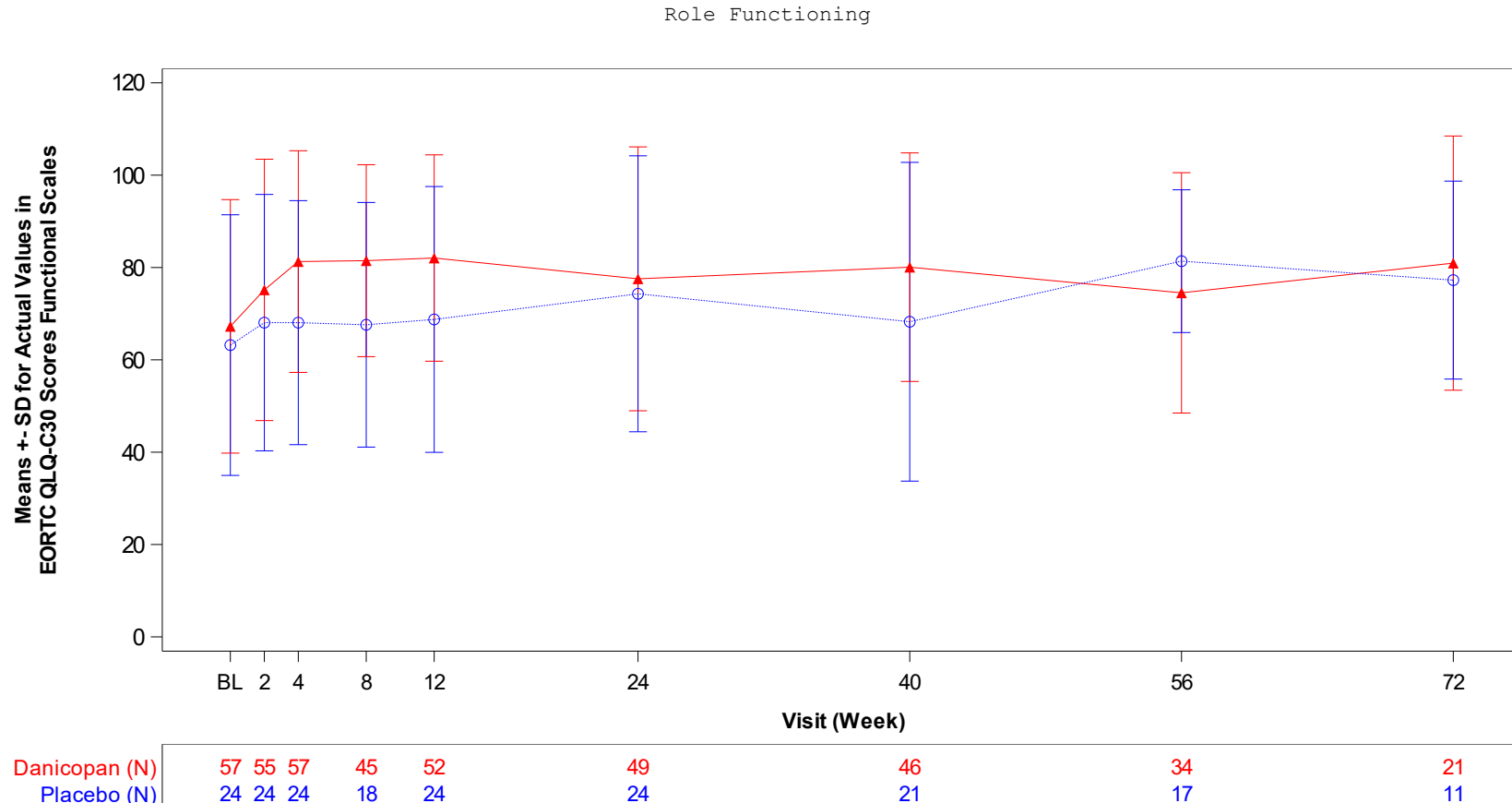
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Figure GERMANY.2.2.7.3
Plots of Mean \pm SD for Actual Values in EORTC QLQ-C30 Scores Functional Scales During 72 Weeks
Modified Randomized Set



Baseline is defined as the last non-missing value prior to the first dose of study drug.
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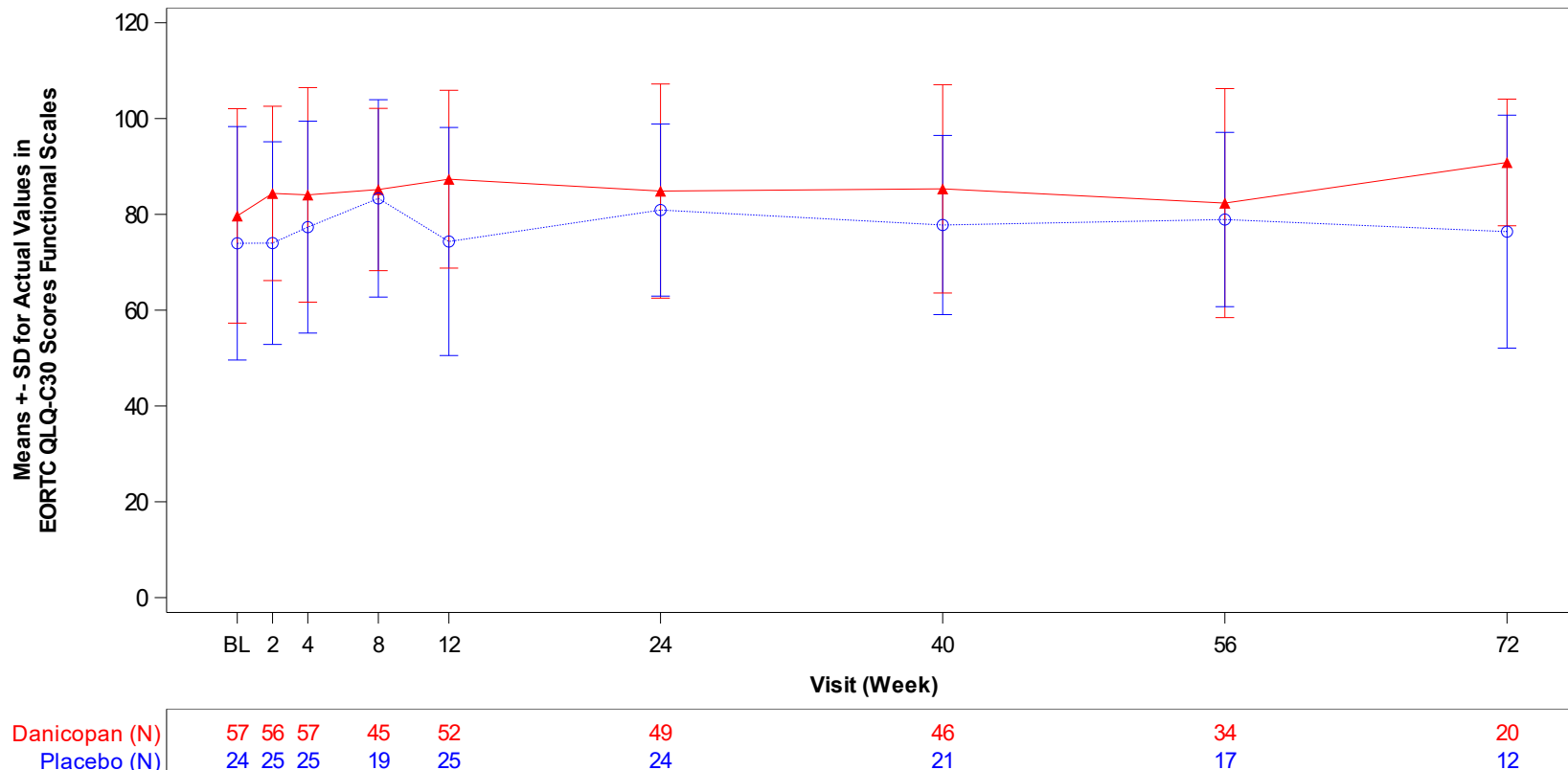
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Figure GERMANY.2.2.7.3
Plots of Mean \pm SD for Actual Values in EORTC QLQ-C30 Scores Functional Scales During 72 Weeks
Modified Randomized Set

Emotional Functioning



Baseline is defined as the last non-missing value prior to the first dose of study drug.

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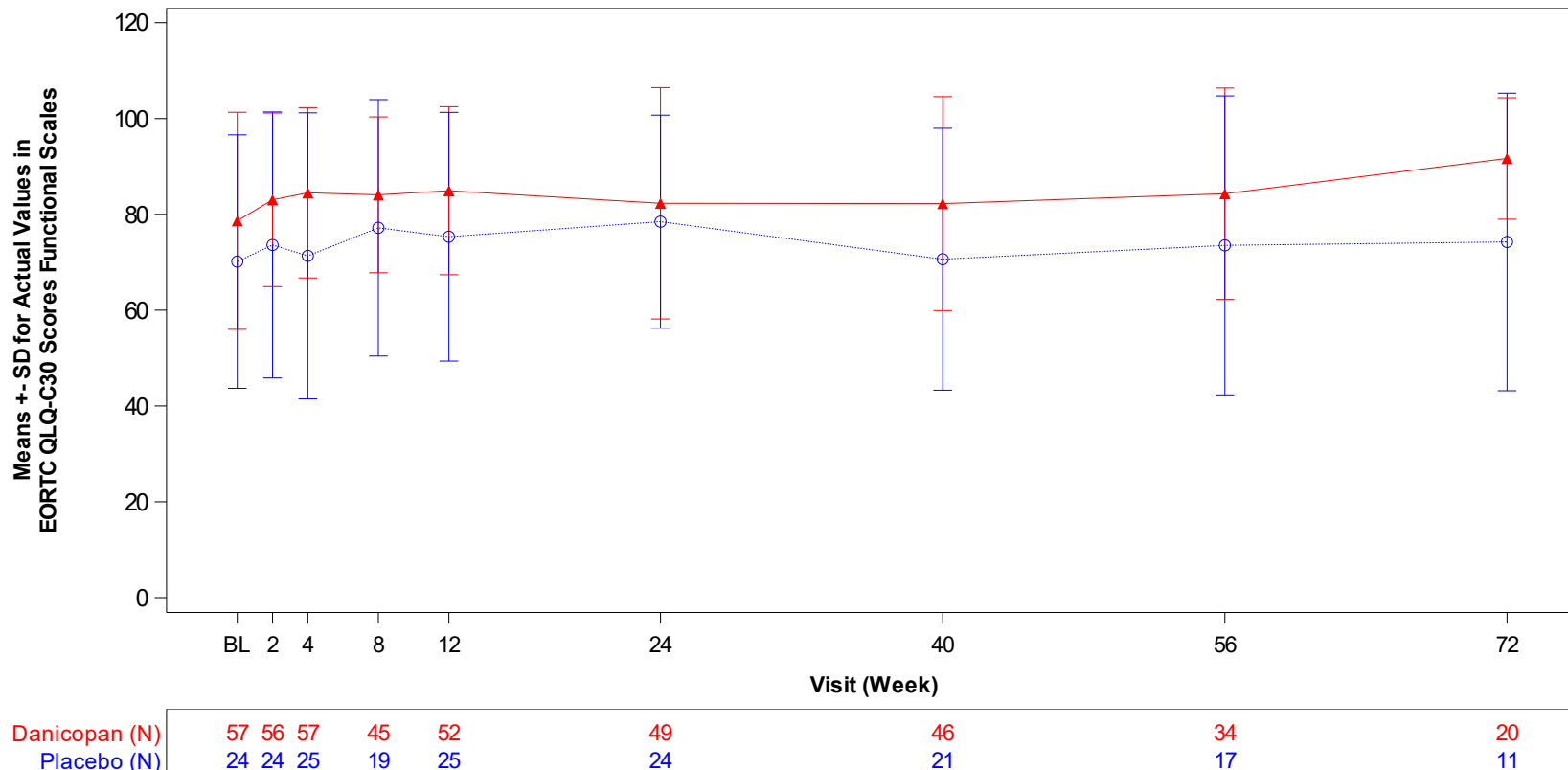
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Figure GERMANY.2.2.7.3
Plots of Mean \pm SD for Actual Values in EORTC QLQ-C30 Scores Functional Scales During 72 Weeks
Modified Randomized Set

Cognitive Functioning



Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Source: ADaM.ADQS

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Data cutoff date of 31Mar2023

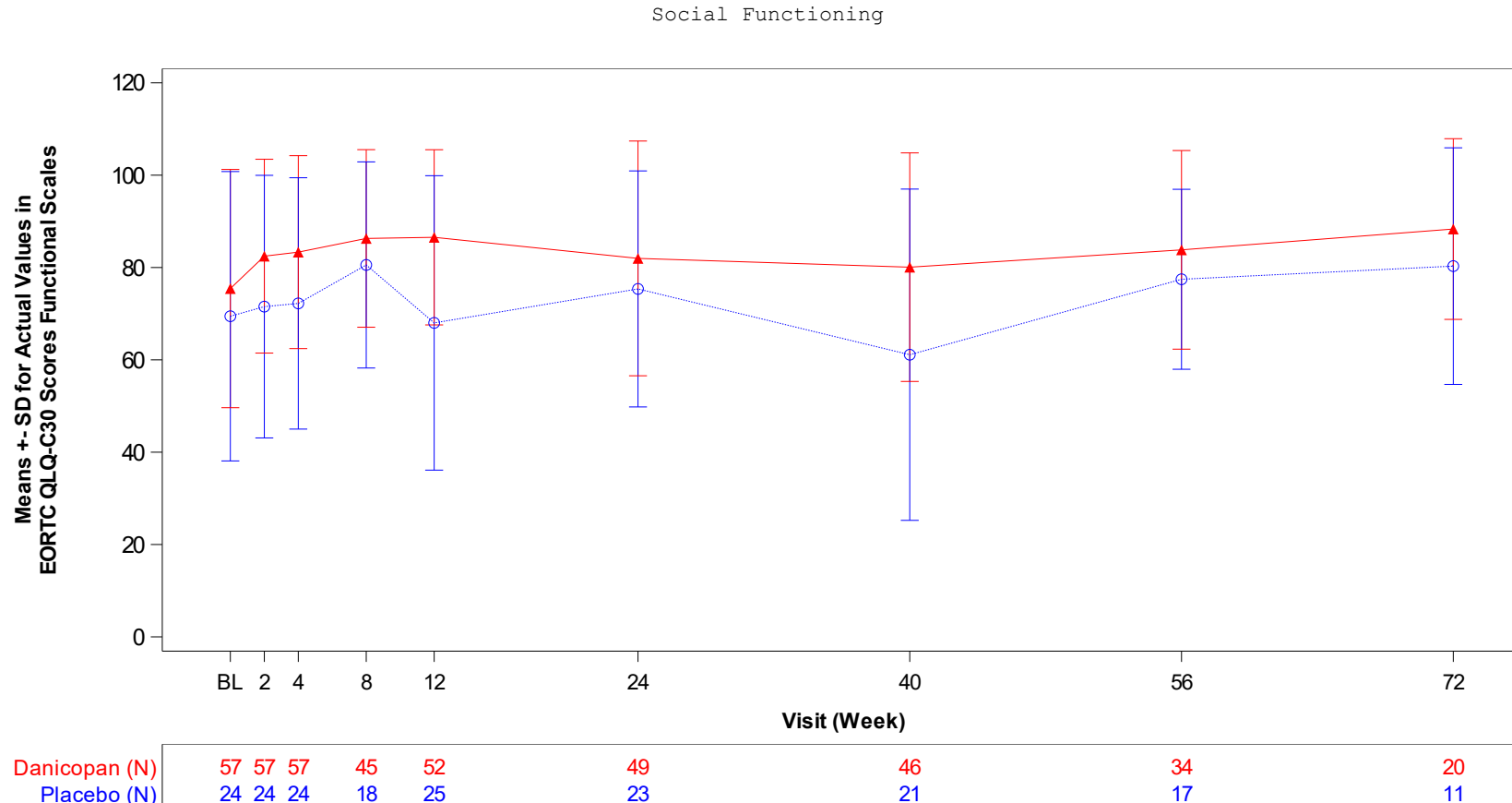
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Figure GERMANY.2.2.7.3
Plots of Mean \pm SD for Actual Values in EORTC QLQ-C30 Scores Functional Scales During 72 Weeks
Modified Randomized Set



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Data cutoff date of 31Mar2023
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Table GERMANY.2.5.10.1
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.24 (2.089)	2.47 (3.247)	3.77 (3.787)	0.3224
	95% CI for LS MEAN	(2.08, 10.40)	(-3.99, 8.93)	(-3.77, 11.31)	
Week 4	LS Mean (SEM)	9.16 (2.110)	-0.90 (3.226)	10.06 (3.786)	0.0096
	95% CI for LS MEAN	(4.96, 13.36)	(-7.32, 5.53)	(2.52, 17.60)	
Week 8	LS Mean (SEM)	8.46 (2.873)	-1.81 (4.470)	10.27 (5.263)	0.0549
	95% CI for LS MEAN	(2.74, 14.19)	(-10.72, 7.10)	(-0.22, 20.77)	
Week 12	LS Mean (SEM)	11.31 (2.084)	6.05 (3.118)	5.26 (3.679)	0.1571
	95% CI for LS MEAN	(7.16, 15.46)	(-0.17, 12.27)	(-2.08, 12.60)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). An Unstructured covariance structure was used.

P-value is from difference of LSM from MMRM model.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.1
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.86 (1.515)	-1.47 (2.340)	7.33 (2.733)	0.0089
	95% CI for LS MEAN	(2.85, 8.88)	(-6.13, 3.19)	(1.89, 12.78)	
Week 4	LS Mean (SEM)	10.98 (1.819)	-1.62 (2.780)	12.60 (3.282)	0.0003
	95% CI for LS MEAN	(7.36, 14.60)	(-7.16, 3.91)	(6.06, 19.13)	
Week 8	LS Mean (SEM)	8.87 (2.030)	-2.20 (3.128)	11.08 (3.694)	0.0037
	95% CI for LS MEAN	(4.83, 12.92)	(-8.44, 4.03)	(3.71, 18.44)	
Week 12	LS Mean (SEM)	10.59 (1.831)	-2.18 (2.755)	12.77 (3.268)	0.0002
	95% CI for LS MEAN	(6.95, 14.23)	(-7.67, 3.31)	(6.26, 19.28)	

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P-value is from difference of LSM from MMRM model.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:47:45

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.1
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	8.10 (2.909)	1.57 (4.583)	6.53 (5.333)	0.2246
	95% CI for LS MEAN	(2.30, 13.90)	(-7.57, 10.70)	(-4.10, 17.17)	
Week 4	LS Mean (SEM)	13.82 (2.998)	0.62 (4.668)	13.20 (5.462)	0.0181
	95% CI for LS MEAN	(7.85, 19.79)	(-8.68, 9.92)	(2.31, 24.08)	
Week 8	LS Mean (SEM)	13.39 (3.121)	-6.53 (4.949)	19.92 (5.758)	0.0010
	95% CI for LS MEAN	(7.15, 19.63)	(-16.42, 3.36)	(8.41, 31.44)	
Week 12	LS Mean (SEM)	13.40 (3.100)	2.80 (4.736)	10.61 (5.576)	0.0611
	95% CI for LS MEAN	(7.23, 19.57)	(-6.64, 12.23)	(-0.51, 21.72)	

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P-value is from difference of LSM from MMRM model.

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Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.1
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.37 (1.717)	-0.11 (2.665)	5.48 (3.102)	0.0811
	95% CI for LS MEAN	(1.95, 8.79)	(-5.42, 5.19)	(-0.69, 11.66)	
Week 4	LS Mean (SEM)	4.93 (2.252)	0.85 (3.460)	4.07 (4.080)	0.3212
	95% CI for LS MEAN	(0.44, 9.41)	(-6.03, 7.74)	(-4.05, 12.20)	
Week 8	LS Mean (SEM)	6.26 (2.068)	3.36 (3.232)	2.90 (3.771)	0.4445
	95% CI for LS MEAN	(2.13, 10.38)	(-3.09, 9.80)	(-4.63, 10.43)	
Week 12	LS Mean (SEM)	7.50 (1.889)	-1.23 (2.825)	8.73 (3.338)	0.0108
	95% CI for LS MEAN	(3.74, 11.26)	(-6.86, 4.40)	(2.08, 15.38)	

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P-value is from difference of LSM from MMRM model.

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Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.1
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.15 (2.208)	-0.18 (3.416)	5.34 (4.006)	0.1867
	95% CI for LS MEAN	(0.76, 9.55)	(-6.98, 6.61)	(-2.64, 13.31)	
Week 4	LS Mean (SEM)	6.41 (2.394)	-2.66 (3.665)	9.07 (4.324)	0.0391
	95% CI for LS MEAN	(1.64, 11.17)	(-9.96, 4.63)	(0.47, 17.68)	
Week 8	LS Mean (SEM)	7.20 (2.189)	1.31 (3.403)	5.89 (3.978)	0.1426
	95% CI for LS MEAN	(2.84, 11.56)	(-5.47, 8.08)	(-2.03, 13.82)	
Week 12	LS Mean (SEM)	6.93 (2.138)	1.50 (3.181)	5.43 (3.769)	0.1539
	95% CI for LS MEAN	(2.68, 11.19)	(-4.84, 7.84)	(-2.08, 12.94)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). An Unstructured covariance structure was used.

P-value is from difference of LSM from MMRM model.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.1
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning

Visit	Statistics	Danicopan (N = 57)	Placebo (N = 24)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	7.50 (2.520)	-3.21 (3.934)	10.71 (4.589)	0.0225
	95% CI for LS MEAN	(2.47, 12.52)	(-11.05, 4.63)	(1.56, 19.86)	
Week 4	LS Mean (SEM)	8.37 (2.465)	-1.79 (3.809)	10.16 (4.457)	0.0257
	95% CI for LS MEAN	(3.46, 13.29)	(-9.38, 5.81)	(1.27, 19.05)	
Week 8	LS Mean (SEM)	10.63 (2.722)	0.13 (4.295)	10.50 (4.995)	0.0402
	95% CI for LS MEAN	(5.18, 16.09)	(-8.47, 8.73)	(0.49, 20.52)	
Week 12	LS Mean (SEM)	10.24 (2.641)	-5.26 (3.952)	15.49 (4.669)	0.0014
	95% CI for LS MEAN	(4.98, 15.49)	(-13.13, 2.62)	(6.19, 24.80)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL). An Unstructured covariance structure was used.

P-value is from difference of LSM from MMRM model.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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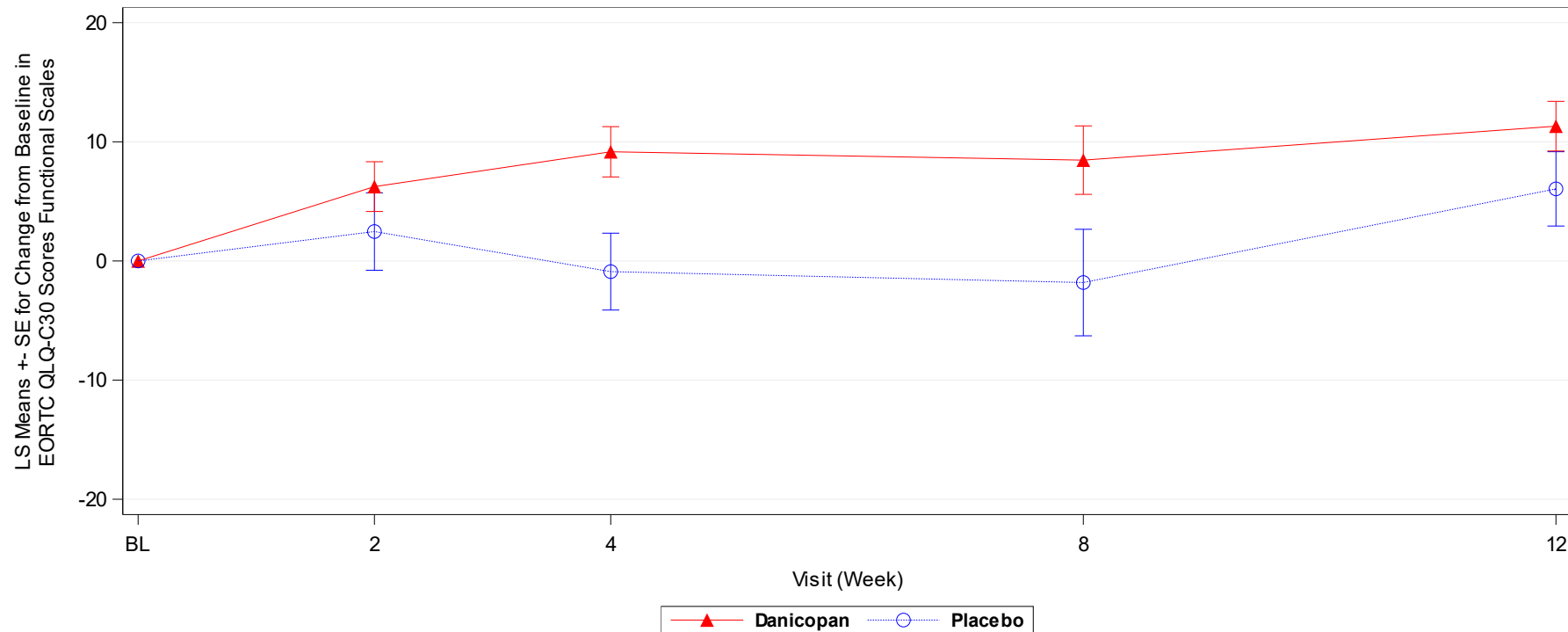
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Figure GERMANY.2.2.7.1
Plots of LS Means \pm SE for Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During 12-Week of Treatment Period 1
Modified Randomized Set

Global Health Status/QoL



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

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Source: ADaM.ADSL, ADaM.ADQS

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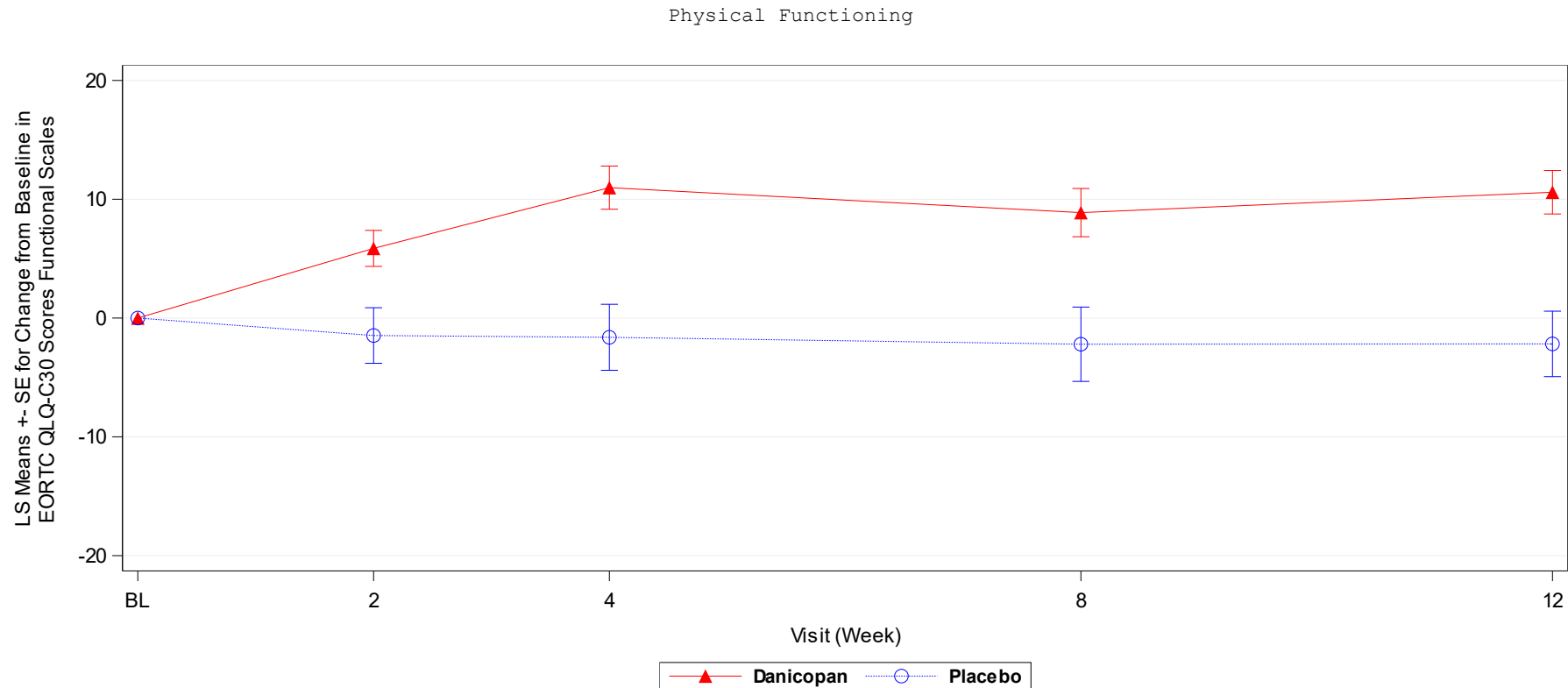
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Figure GERMANY.2.2.7.1
Plots of LS Means +- SE for Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During 12-Week of Treatment Period 1
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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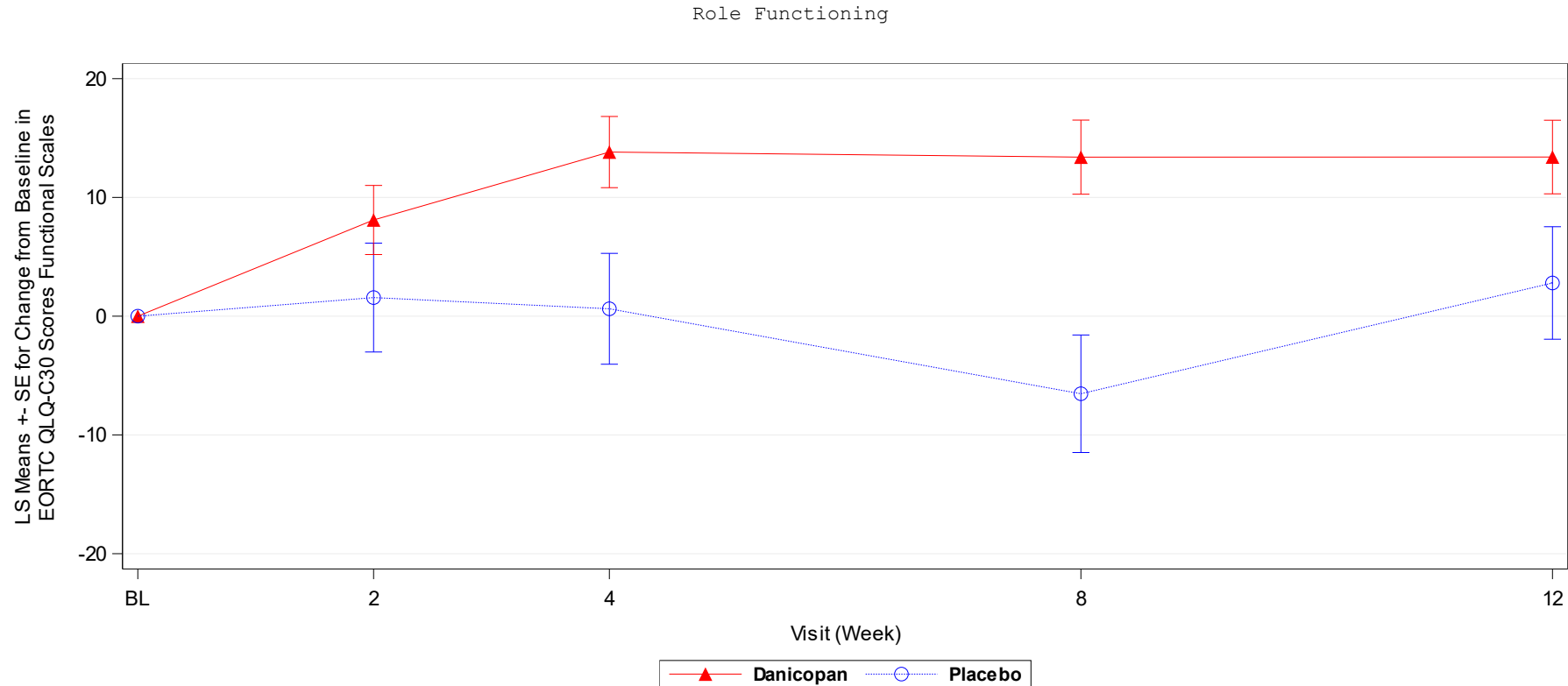
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Figure GERMANY.2.2.7.1
Plots of LS Means \pm SE for Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During 12-Week of Treatment Period 1
Modified Randomized Set



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Source: ADaM.ADSL, ADaM.ADQS

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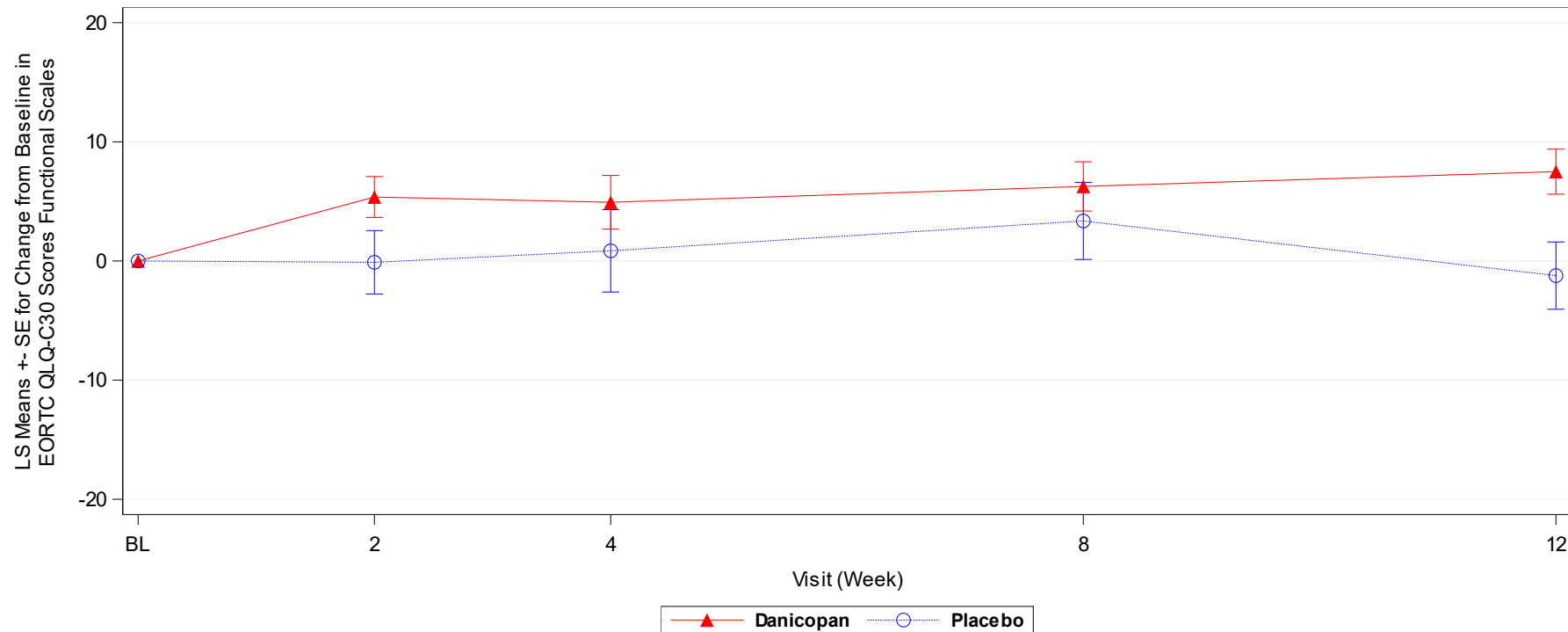
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Figure GERMANY.2.2.7.1
Plots of LS Means \pm SE for Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During 12-Week of Treatment Period 1
Modified Randomized Set

Emotional Functioning



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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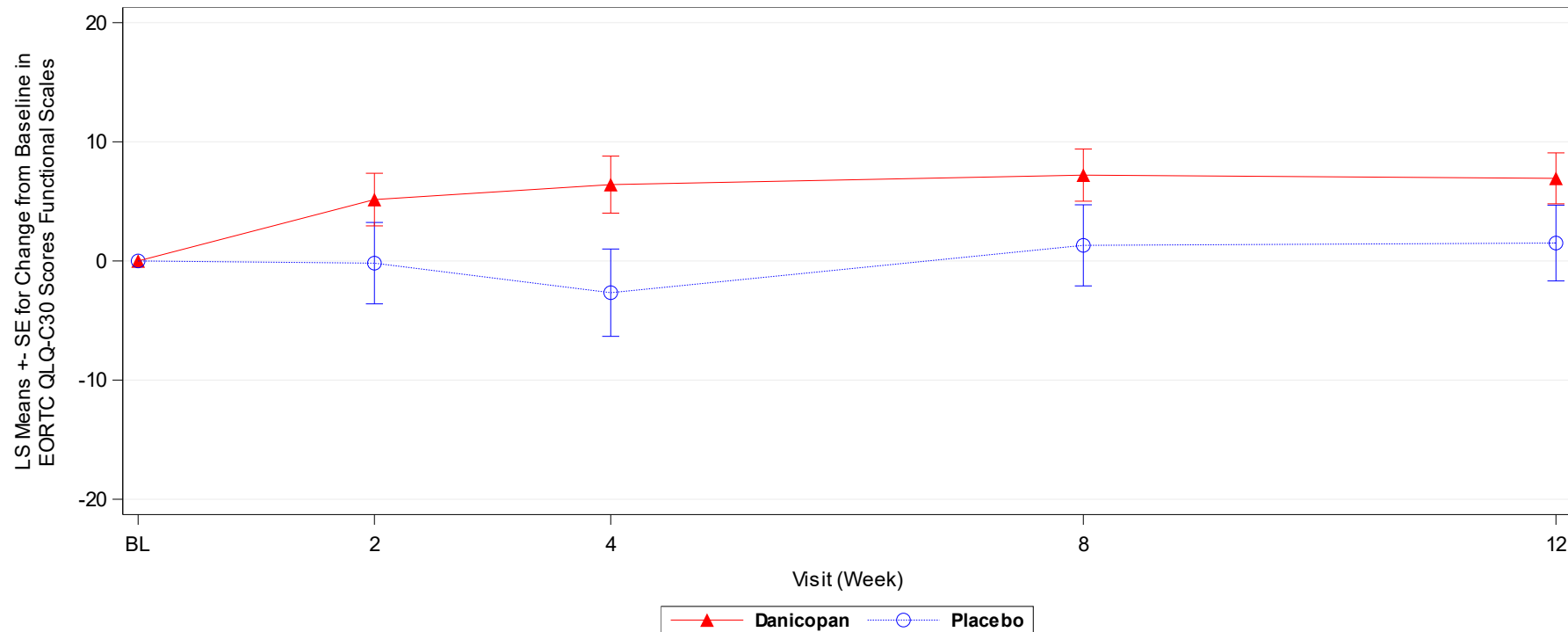
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Figure GERMANY.2.2.7.1
Plots of LS Means \pm SE for Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During 12-Week of Treatment Period 1
Modified Randomized Set

Cognitive Functioning



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Source: ADaM.ADSL, ADaM.ADQS

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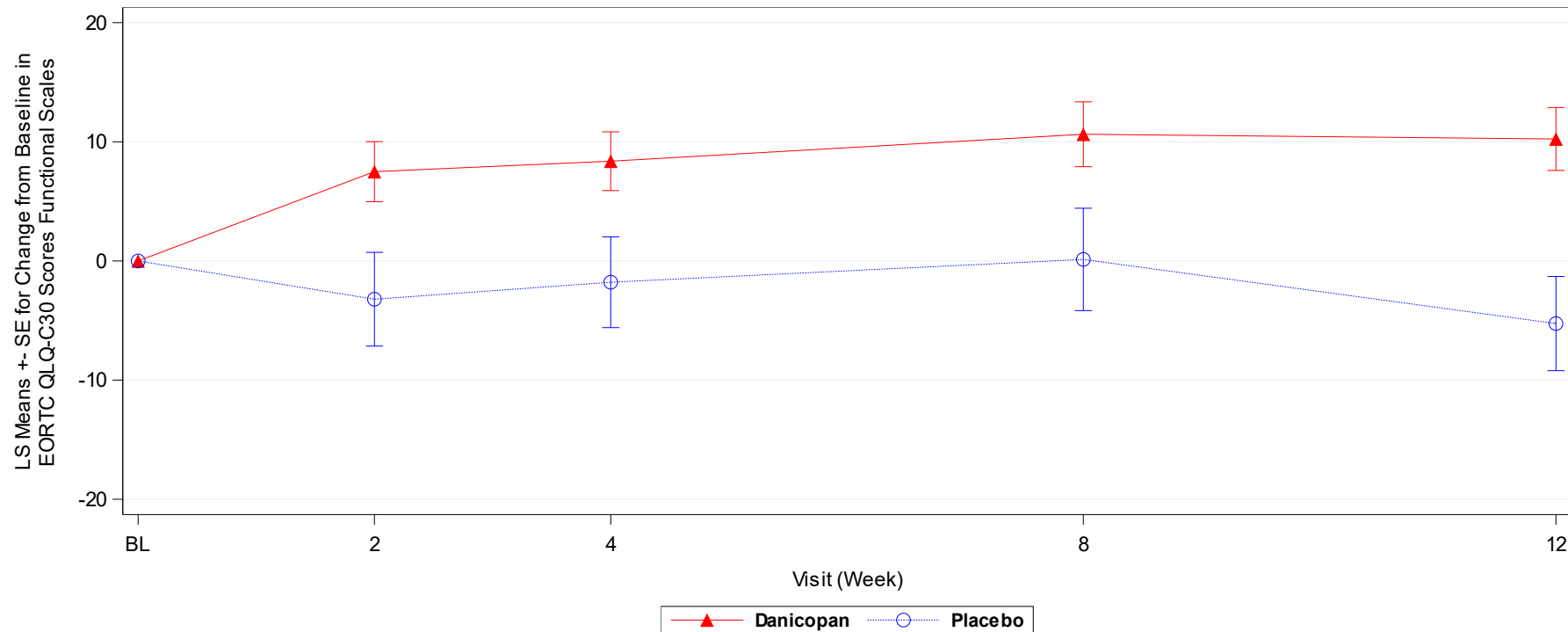
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Figure GERMANY.2.2.7.1
Plots of LS Means \pm SE for Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During 12-Week of Treatment Period 1
Modified Randomized Set

Social Functioning



LS=Least squares; SE=Standard error of the mean.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

LSmeans and SE are from Mixed Model for Repeated Measures (MMRM).

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

2 Gesundheitsbezogene Lebensqualität

2.1 Gesundheitsbezogene Lebensqualität anhand des EORTC QLQ-C30

2.1.2 Veränderung des EORTC QLQ-C30 Gesundheitsstatus und der Funktionsskalen im Vergleich zu Baseline

2.1.2.1 Subgruppenanalysen – Veränderung des EORTC QLQ-C30 Gesundheitsstatus und der Funktionsskalen im Vergleich zu Baseline

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Table GERMANY.2.5.11.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Global Health Status/QoL

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean (SD)	56.88 (21.854)		63.89 (16.137)		58.85 (20.406)	
Median	66.67		66.67		66.67	
Min, Max	16.7, 91.7		41.7, 91.7		16.7, 91.7	
Week 2						
n	23	23	8	8		
Mean (SD)	67.03 (16.944)	10.14 (12.801)	69.79 (12.550)	6.25 (19.796)		
Median	66.67	8.33	66.67	4.17		
Min, Max	33.3, 91.7	-8.3, 41.7	50.0, 83.3	-16.7, 33.3		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.11.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Global Health Status/QoL

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	23	23	9	9		
Mean (SD)	65.58 (18.516)	8.70 (15.175)	65.74 (18.840)	1.85 (14.299)		
Median	66.67	0.00	66.67	0.00		
Min, Max	25.0, 100.0	-8.3, 50.0	33.3, 100.0	-16.7, 25.0		
Week 8						
n	19	19	7	7		
Mean (SD)	65.35 (22.950)	5.26 (15.518)	75.00 (8.333)	9.52 (18.276)		
Median	66.67	8.33	75.00	16.67		
Min, Max	16.7, 100.0	-41.7, 33.3	66.7, 83.3	-16.7, 33.3		

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.11.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Global Health Status/QoL

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	9	9		
Mean (SD)	68.56 (20.401)	12.12 (13.784)	70.37 (17.732)	6.48 (18.993)		
Median	66.67	12.50	66.67	0.00		
Min, Max	33.3, 100.0	-16.7, 33.3	33.3, 91.7	-16.7, 41.7		
Week 24						
n	21	21	9	9		
Mean (SD)	67.46 (23.110)	9.13 (19.348)	71.30 (20.880)	7.41 (17.895)		
Median	66.67	8.33	66.67	8.33		
Min, Max	16.7, 100.0	-33.3, 41.7	33.3, 100.0	-16.7, 41.7		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.11.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Global Health Status/QoL

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	8	8	29	29
Mean (SD)	67.46 (22.033)	9.92 (12.253)	76.04 (15.064)	14.58 (14.605)	69.83 (20.463)	11.21 (12.848)
Median	75.00	8.33	79.17	16.67	75.00	16.67
Min, Max	33.3, 100.0	-16.7, 33.3	50.0, 100.0	-16.7, 33.3	33.3, 100.0	-16.7, 33.3
Week 56						
n	17	17	5	5	22	22
Mean (SD)	68.14 (17.978)	8.82 (17.298)	70.00 (13.944)	3.33 (17.280)	68.56 (16.851)	7.58 (17.041)
Median	66.67	0.00	66.67	0.00	66.67	0.00
Min, Max	33.3, 100.0	-16.7, 41.7	50.0, 83.3	-16.7, 25.0	33.3, 100.0	-16.7, 41.7

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.11.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Global Health Status/QoL

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	2	2	10	10
Mean (SD)	72.92 (12.400)	14.58 (19.288)	66.67 (23.570)	4.17 (29.463)	71.67 (13.721)	12.50 (20.127)
Median	66.67	8.33	66.67	4.17	66.67	8.33
Min, Max	66.7, 100.0	-8.3, 41.7	50.0, 83.3	-16.7, 25.0	50.0, 100.0	-16.7, 41.7
Week 88						
n	6	6	2	2	8	8
Mean (SD)	70.83 (16.457)	8.33 (10.541)	66.67 (23.570)	4.17 (29.463)	69.79 (16.629)	7.29 (14.391)
Median	75.00	12.50	66.67	4.17	75.00	12.50
Min, Max	41.7, 83.3	-8.3, 16.7	50.0, 83.3	-16.7, 25.0	41.7, 83.3	-16.7, 25.0

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Table GERMANY.2.5.11.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Global Health Status/QoL

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	66.67 (NA)	0.00 (NA)			66.67 (NA)	0.00 (NA)
Median	66.67	0.00			66.67	0.00
Min, Max	66.7, 66.7	0.0, 0.0			66.7, 66.7	0.0, 0.0

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Physical Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean (SD)	75.36 (19.533)		74.07 (13.922)		75.00 (17.921)	
Median	80.00		73.33		76.67	
Min, Max	33.3, 100.0		60.0, 100.0		33.3, 100.0	
Week 2						
n	22	22	8	8		
Mean (SD)	83.64 (17.545)	9.09 (13.885)	76.67 (13.333)	0.83 (7.507)		
Median	86.67	6.67	70.00	0.00		
Min, Max	26.7, 100.0	-13.3, 46.7	66.7, 100.0	-6.7, 13.3		

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Modified Randomized Set

Physical Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	23	23	9	9		
Mean (SD)	87.83 (12.497)	12.46 (15.479)	74.81 (17.882)	0.74 (10.244)		
Median	86.67	6.67	73.33	0.00		
Min, Max	53.3, 100.0	-6.7, 60.0	46.7, 100.0	-13.3, 13.3		
Week 8						
n	19	19	7	7		
Mean (SD)	85.61 (14.447)	7.37 (11.945)	80.00 (13.333)	4.76 (6.341)		
Median	86.67	6.67	73.33	0.00		
Min, Max	53.3, 100.0	-13.3, 33.3	66.7, 100.0	0.0, 13.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Physical Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	9	9		
Mean (SD)	86.36 (17.636)	10.91 (13.420)	72.59 (18.088)	-1.48 (14.055)		
Median	90.00	6.67	73.33	0.00		
Min, Max	26.7, 100.0	-6.7, 40.0	46.7, 100.0	-33.3, 13.3		
Week 24						
n	21	21	9	9		
Mean (SD)	87.94 (16.414)	12.06 (15.580)	80.74 (20.123)	6.67 (12.472)		
Median	93.33	13.33	86.67	6.67		
Min, Max	40.0, 100.0	-6.7, 60.0	53.3, 100.0	-6.7, 26.7		

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Physical Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	8	8	29	29
Mean (SD)	85.08 (21.072)	10.48 (16.542)	80.00 (22.817)	5.00 (13.214)	83.68 (21.276)	8.97 (15.663)
Median	93.33	13.33	86.67	10.00	86.67	13.33
Min, Max	13.3, 100.0	-20.0, 53.3	40.0, 100.0	-20.0, 20.0	13.3, 100.0	-20.0, 53.3
Week 56						
n	17	17	5	5	22	22
Mean (SD)	86.27 (17.235)	12.55 (16.310)	85.33 (23.286)	4.00 (12.996)	86.06 (18.160)	10.61 (15.757)
Median	93.33	13.33	100.00	0.00	93.33	13.33
Min, Max	46.7, 100.0	-6.7, 60.0	46.7, 100.0	-13.3, 20.0	46.7, 100.0	-13.3, 60.0

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Physical Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	2	2	10	10
Mean (SD)	87.50 (12.567)	17.50 (17.795)	86.67 (18.856)	16.67 (4.714)	87.33 (12.746)	17.33 (15.776)
Median	93.33	13.33	86.67	16.67	93.33	13.33
Min, Max	66.7, 100.0	6.7, 60.0	73.3, 100.0	13.3, 20.0	66.7, 100.0	6.7, 60.0
Week 88						
n	6	6	2	2	8	8
Mean (SD)	87.78 (14.857)	13.33 (11.926)	76.67 (32.998)	6.67 (18.856)	85.00 (18.430)	11.67 (12.724)
Median	90.00	10.00	76.67	6.67	90.00	10.00
Min, Max	60.0, 100.0	0.0, 33.3	53.3, 100.0	-6.7, 20.0	53.3, 100.0	-6.7, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Physical Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	100.00 (NA)	6.67 (NA)			100.00 (NA)	6.67 (NA)
Median	100.00	6.67			100.00	6.67
Min, Max	100.0, 100.0	6.7, 6.7			100.0, 100.0	6.7, 6.7

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Role Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean (SD)	71.01 (28.523)		72.22 (22.048)		71.35 (26.517)	
Median	66.67		66.67		66.67	
Min, Max	0.0, 100.0		33.3, 100.0		0.0, 100.0	
Week 2						
n	22	22	8	8		
Mean (SD)	76.52 (28.011)	6.82 (27.535)	75.00 (17.817)	-2.08 (16.517)		
Median	83.33	0.00	66.67	0.00		
Min, Max	0.0, 100.0	-50.0, 66.7	50.0, 100.0	-33.3, 16.7		

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Role Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	23	23	9	9		
Mean (SD)	82.61 (25.858)	11.59 (29.057)	75.93 (18.840)	3.70 (11.111)		
Median	100.00	0.00	83.33	0.00		
Min, Max	0.0, 100.0	-50.0, 83.3	50.0, 100.0	-16.7, 16.7		
Week 8						
n	19	19	7	7		
Mean (SD)	81.58 (22.835)	7.89 (23.153)	71.43 (15.853)	-9.52 (16.265)		
Median	83.33	0.00	66.67	-16.67		
Min, Max	33.3, 100.0	-33.3, 50.0	50.0, 100.0	-33.3, 16.7		

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Role Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	9	9		
Mean (SD)	81.06 (26.872)	10.61 (22.739)	75.93 (26.498)	3.70 (29.788)		
Median	100.00	0.00	83.33	0.00		
Min, Max	0.0, 100.0	-33.3, 50.0	16.7, 100.0	-33.3, 50.0		
Week 24						
n	21	21	9	9		
Mean (SD)	80.95 (28.031)	10.32 (30.035)	81.48 (17.568)	9.26 (26.498)		
Median	100.00	0.00	83.33	16.67		
Min, Max	0.0, 100.0	-50.0, 83.3	50.0, 100.0	-33.3, 33.3		

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Table GERMANY.2.5.11.2
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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Role Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	8	8	29	29
Mean (SD)	78.57 (28.936)	7.14 (26.652)	77.08 (28.084)	8.33 (19.920)	78.16 (28.208)	7.47 (24.635)
Median	100.00	0.00	83.33	8.33	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 66.7	16.7, 100.0	-16.7, 33.3	0.0, 100.0	-66.7, 66.7
Week 56						
n	17	17	5	5	22	22
Mean (SD)	76.47 (30.080)	7.84 (31.797)	90.00 (14.907)	23.33 (14.907)	79.55 (27.666)	11.36 (29.271)
Median	83.33	0.00	100.00	33.33	91.67	8.33
Min, Max	0.0, 100.0	-83.3, 66.7	66.7, 100.0	0.0, 33.3	0.0, 100.0	-83.3, 66.7

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Role Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	2	2	10	10
Mean (SD)	85.42 (16.517)	27.08 (28.084)	91.67 (11.785)	41.67 (35.355)	86.67 (15.316)	30.00 (28.109)
Median	91.67	25.00	91.67	41.67	91.67	25.00
Min, Max	66.7, 100.0	-16.7, 66.7	83.3, 100.0	16.7, 66.7	66.7, 100.0	-16.7, 66.7
Week 88						
n	6	6	2	2	8	8
Mean (SD)	91.67 (13.944)	25.00 (22.973)	83.33 (23.570)	33.33 (0.000)	89.58 (15.269)	27.08 (19.796)
Median	100.00	25.00	83.33	33.33	100.00	33.33
Min, Max	66.7, 100.0	0.0, 50.0	66.7, 100.0	33.3, 33.3	66.7, 100.0	0.0, 50.0

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Role Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	100.00 (NA)	0.00 (NA)			100.00 (NA)	0.00 (NA)
Median	100.00	0.00			100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0			100.0, 100.0	0.0, 0.0

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Emotional Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean (SD)	80.43 (24.180)		87.04 (10.300)		82.29 (21.246)	
Median	83.33		83.33		83.33	
Min, Max	0.0, 100.0		66.7, 100.0		0.0, 100.0	
Week 2						
n	23	23	8	8		
Mean (SD)	84.78 (18.232)	4.35 (14.405)	84.38 (9.383)	-3.13 (12.550)		
Median	91.67	0.00	83.33	-8.33		
Min, Max	33.3, 100.0	-16.7, 33.3	75.0, 100.0	-16.7, 25.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Emotional Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	23	23	9	9		
Mean (SD)	86.96 (16.443)	6.52 (13.521)	93.52 (8.099)	6.48 (13.679)		
Median	91.67	0.00	100.00	0.00		
Min, Max	33.3, 100.0	-8.3, 41.7	83.3, 100.0	-8.3, 33.3		
Week 8						
n	19	19	7	7		
Mean (SD)	85.53 (13.845)	3.95 (10.529)	94.05 (7.927)	5.95 (16.467)		
Median	83.33	0.00	100.00	0.00		
Min, Max	66.7, 100.0	-25.0, 25.0	83.3, 100.0	-8.3, 33.3		

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Emotional Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	9	9		
Mean (SD)	85.61 (20.922)	4.55 (10.828)	87.96 (17.732)	0.93 (20.601)		
Median	100.00	0.00	100.00	0.00		
Min, Max	16.7, 100.0	-16.7, 33.3	50.0, 100.0	-33.3, 33.3		
Week 24						
n	21	21	9	9		
Mean (SD)	87.70 (22.457)	5.56 (10.971)	91.67 (10.206)	4.63 (17.236)		
Median	100.00	0.00	100.00	0.00		
Min, Max	8.3, 100.0	-16.7, 33.3	75.0, 100.0	-16.7, 33.3		

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Emotional Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	8	8	29	29
Mean (SD)	87.30 (19.476)	6.75 (11.966)	86.46 (17.216)	0.00 (20.893)	87.07 (18.579)	4.89 (14.860)
Median	91.67	8.33	95.83	-4.17	91.67	8.33
Min, Max	16.7, 100.0	-16.7, 33.3	58.3, 100.0	-25.0, 33.3	16.7, 100.0	-25.0, 33.3
Week 56						
n	17	17	5	5	22	22
Mean (SD)	85.78 (20.574)	6.37 (16.804)	90.00 (14.907)	6.67 (22.361)	86.74 (19.186)	6.44 (17.618)
Median	100.00	0.00	100.00	16.67	100.00	4.17
Min, Max	33.3, 100.0	-25.0, 33.3	66.7, 100.0	-16.7, 33.3	33.3, 100.0	-25.0, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
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Emotional Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	2	2	10	10
Mean (SD)	91.67 (10.911)	10.42 (15.905)	100.00 (0.000)	25.00 (11.785)	93.33 (10.244)	13.33 (15.811)
Median	95.83	4.17	100.00	25.00	100.00	12.50
Min, Max	75.0, 100.0	-8.3, 33.3	100.0, 100.0	16.7, 33.3	75.0, 100.0	-8.3, 33.3
Week 88						
n	6	6	2	2	8	8
Mean (SD)	93.06 (9.742)	8.33 (11.785)	100.00 (0.000)	25.00 (11.785)	94.79 (8.839)	12.50 (13.363)
Median	95.83	8.33	100.00	25.00	100.00	12.50
Min, Max	75.0, 100.0	-8.3, 25.0	100.0, 100.0	16.7, 33.3	75.0, 100.0	-8.3, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Emotional Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	100.00 (NA)	0.00 (NA)			100.00 (NA)	0.00 (NA)
Median	100.00	0.00			100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0			100.0, 100.0	0.0, 0.0

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Cognitive Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean (SD)	76.81 (24.484)		87.04 (13.889)		79.69 (22.294)	
Median	83.33		83.33		83.33	
Min, Max	16.7, 100.0		66.7, 100.0		16.7, 100.0	
Week 2						
n	23	23	8	8		
Mean (SD)	82.61 (17.025)	5.80 (17.844)	87.50 (14.773)	2.08 (20.774)		
Median	83.33	0.00	91.67	0.00		
Min, Max	50.0, 100.0	-16.7, 66.7	66.7, 100.0	-33.3, 33.3		

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Table GERMANY.2.5.11.2
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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Cognitive Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	23	23	9	9		
Mean (SD)	84.78 (17.345)	7.97 (14.966)	88.89 (14.434)	1.85 (25.610)		
Median	83.33	0.00	100.00	0.00		
Min, Max	33.3, 100.0	-16.7, 50.0	66.7, 100.0	-33.3, 33.3		
Week 8						
n	19	19	7	7		
Mean (SD)	82.46 (15.188)	5.26 (14.754)	90.48 (13.113)	4.76 (20.893)		
Median	83.33	0.00	100.00	0.00		
Min, Max	50.0, 100.0	-16.7, 33.3	66.7, 100.0	-33.3, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Cognitive Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	9	9		
Mean (SD)	82.58 (19.570)	6.82 (15.135)	87.04 (13.889)	0.00 (20.412)		
Median	83.33	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-16.7, 50.0	66.7, 100.0	-33.3, 33.3		
Week 24						
n	21	21	9	9		
Mean (SD)	77.78 (27.046)	1.59 (18.185)	88.89 (14.434)	1.85 (19.444)		
Median	83.33	0.00	100.00	0.00		
Min, Max	0.0, 100.0	-33.3, 50.0	66.7, 100.0	-33.3, 33.3		

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Cognitive Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	8	8	29	29
Mean (SD)	79.37 (21.670)	4.76 (17.593)	83.33 (15.430)	-4.17 (21.362)	80.46 (19.955)	2.30 (18.753)
Median	83.33	0.00	83.33	0.00	83.33	0.00
Min, Max	33.3, 100.0	-50.0, 50.0	66.7, 100.0	-33.3, 33.3	33.3, 100.0	-50.0, 50.0
Week 56						
n	17	17	5	5	22	22
Mean (SD)	80.39 (23.000)	5.88 (17.620)	86.67 (13.944)	3.33 (21.731)	81.82 (21.150)	5.30 (18.102)
Median	83.33	0.00	83.33	0.00	83.33	0.00
Min, Max	33.3, 100.0	-33.3, 50.0	66.7, 100.0	-16.7, 33.3	33.3, 100.0	-33.3, 50.0

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
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Cognitive Functioning

Male

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	2	2	10	10
Mean (SD)	87.50 (14.773)	8.33 (17.817)	100.00 (0.000)	16.67 (23.570)	90.00 (14.055)	10.00 (17.916)
Median	91.67	0.00	100.00	16.67	100.00	0.00
Min, Max	66.7, 100.0	0.0, 50.0	100.0, 100.0	0.0, 33.3	66.7, 100.0	0.0, 50.0
Week 88						
n	6	6	2	2	8	8
Mean (SD)	97.22 (6.804)	8.33 (13.944)	91.67 (11.785)	8.33 (35.355)	95.83 (7.715)	8.33 (17.817)
Median	100.00	0.00	91.67	8.33	100.00	0.00
Min, Max	83.3, 100.0	0.0, 33.3	83.3, 100.0	-16.7, 33.3	83.3, 100.0	-16.7, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Cognitive Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	100.00 (NA)	0.00 (NA)			100.00 (NA)	0.00 (NA)
Median	100.00	0.00			100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0			100.0, 100.0	0.0, 0.0

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Social Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	23		9		32	
Mean (SD)	80.43 (30.423)		79.63 (18.215)		80.21 (27.251)	
Median	100.00		83.33		91.67	
Min, Max	0.0, 100.0		50.0, 100.0		0.0, 100.0	
Week 2						
n	23	23	8	8		
Mean (SD)	84.78 (19.406)	4.35 (29.822)	81.25 (16.517)	4.17 (14.773)		
Median	100.00	0.00	75.00	0.00		
Min, Max	50.0, 100.0	-50.0, 83.3	66.7, 100.0	-16.7, 33.3		

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Social Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	23	23	9	9		
Mean (SD)	88.41 (16.233)	7.97 (25.559)	85.19 (19.444)	5.56 (16.667)		
Median	100.00	0.00	100.00	0.00		
Min, Max	50.0, 100.0	-33.3, 83.3	50.0, 100.0	-16.7, 33.3		
Week 8						
n	19	19	7	7		
Mean (SD)	85.09 (20.709)	0.88 (19.621)	80.95 (14.996)	2.38 (17.817)		
Median	100.00	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-50.0, 33.3	66.7, 100.0	-16.7, 33.3		

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Social Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	9	9		
Mean (SD)	87.12 (21.164)	6.06 (22.150)	83.33 (18.634)	3.70 (18.215)		
Median	100.00	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-33.3, 83.3	50.0, 100.0	-16.7, 33.3		
Week 24						
n	21	21	9	9		
Mean (SD)	86.51 (24.506)	6.35 (27.627)	81.48 (15.466)	1.85 (21.155)		
Median	100.00	0.00	83.33	0.00		
Min, Max	0.0, 100.0	-33.3, 83.3	66.7, 100.0	-33.3, 33.3		

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Social Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	8	8	29	29
Mean (SD)	84.92 (21.019)	3.17 (31.010)	66.67 (33.333)	-10.42 (47.923)	79.89 (25.735)	-0.57 (36.045)
Median	100.00	0.00	83.33	0.00	83.33	0.00
Min, Max	33.3, 100.0	-50.0, 83.3	0.0, 100.0	-100.0, 33.3	0.0, 100.0	-100.0, 83.3
Week 56						
n	17	17	5	5	22	22
Mean (SD)	86.27 (23.000)	8.82 (31.246)	83.33 (11.785)	6.67 (19.003)	85.61 (20.763)	8.33 (28.522)
Median	100.00	0.00	83.33	0.00	100.00	0.00
Min, Max	33.3, 100.0	-50.0, 83.3	66.7, 100.0	-16.7, 33.3	33.3, 100.0	-50.0, 83.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Social Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	2	2	10	10
Mean (SD)	91.67 (17.817)	25.00 (29.547)	100.00 (0.000)	16.67 (23.570)	93.33 (16.102)	23.33 (27.442)
Median	100.00	16.67	100.00	16.67	100.00	16.67
Min, Max	50.0, 100.0	0.0, 83.3	100.0, 100.0	0.0, 33.3	50.0, 100.0	0.0, 83.3
Week 88						
n	6	6	2	2	8	8
Mean (SD)	94.44 (13.608)	22.22 (31.032)	83.33 (23.570)	0.00 (47.140)	91.67 (15.430)	16.67 (33.333)
Median	100.00	16.67	83.33	0.00	100.00	16.67
Min, Max	66.7, 100.0	0.0, 83.3	66.7, 100.0	-33.3, 33.3	66.7, 100.0	-33.3, 83.3

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Table GERMANY.2.5.11.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Social Functioning

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	100.00 (NA)	0.00 (NA)			100.00 (NA)	0.00 (NA)
Median	100.00	0.00			100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0			100.0, 100.0	0.0, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Global Health Status/QoL

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		15		49	
Mean (SD)	59.31 (19.219)		50.00 (16.667)		56.46 (18.809)	
Median	54.17		50.00		50.00	
Min, Max	33.3, 100.0		16.7, 75.0		16.7, 100.0	
Week 2						
n	34	34	17	15		
Mean (SD)	61.76 (20.630)	2.45 (19.627)	52.94 (14.416)	1.67 (18.687)		
Median	66.67	0.00	50.00	0.00		
Min, Max	16.7, 100.0	-50.0, 33.3	25.0, 83.3	-25.0, 50.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Global Health Status/QoL

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	34	34	16	15		
Mean (SD)	67.65 (20.388)	8.33 (17.648)	50.52 (21.402)	-1.67 (18.148)		
Median	66.67	0.00	50.00	0.00		
Min, Max	25.0, 100.0	-16.7, 41.7	25.0, 83.3	-41.7, 41.7		
Week 8						
n	26	26	12	11		
Mean (SD)	68.59 (21.513)	11.54 (26.570)	49.31 (23.154)	-3.03 (23.056)		
Median	66.67	20.83	50.00	0.00		
Min, Max	16.7, 100.0	-58.3, 50.0	16.7, 83.3	-33.3, 41.7		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Global Health Status/QoL

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	16	15		
Mean (SD)	70.83 (17.880)	12.22 (16.914)	57.81 (15.356)	6.67 (18.687)		
Median	66.67	12.50	50.00	16.67		
Min, Max	33.3, 100.0	-16.7, 41.7	33.3, 83.3	-25.0, 33.3		
Week 24						
n	28	28	15	14		
Mean (SD)	69.94 (16.408)	10.71 (15.853)	63.33 (17.762)	12.50 (13.377)		
Median	66.67	16.67	66.67	12.50		
Min, Max	33.3, 100.0	-16.7, 33.3	16.7, 83.3	-8.3, 33.3		

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Modified Randomized Set

Global Health Status/QoL

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	25	25	13	13	38	38
Mean (SD)	69.67 (17.658)	10.33 (18.985)	54.49 (23.964)	1.28 (18.586)	64.47 (21.018)	7.24 (19.099)
Median	66.67	8.33	50.00	0.00	66.67	4.17
Min, Max	33.3, 100.0	-41.7, 33.3	16.7, 100.0	-33.3, 33.3	16.7, 100.0	-41.7, 33.3
Week 56						
n	17	17	12	11	29	28
Mean (SD)	65.69 (19.738)	5.88 (15.525)	57.64 (22.321)	0.00 (22.973)	62.36 (20.848)	3.57 (18.624)
Median	66.67	0.00	58.33	0.00	66.67	0.00
Min, Max	33.3, 100.0	-16.7, 41.7	8.3, 83.3	-25.0, 41.7	8.3, 100.0	-25.0, 41.7

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Global Health Status/QoL

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	10	9	22	21
Mean (SD)	67.36 (17.929)	3.47 (24.479)	61.67 (23.960)	1.85 (25.947)	64.77 (20.563)	2.78 (24.485)
Median	66.67	4.17	66.67	0.00	66.67	0.00
Min, Max	33.3, 91.7	-50.0, 41.7	16.7, 91.7	-33.3, 50.0	16.7, 91.7	-50.0, 50.0
Week 88						
n	8	8	6	6	14	14
Mean (SD)	77.08 (14.605)	8.33 (19.416)	59.72 (23.814)	4.17 (25.139)	69.64 (20.309)	6.55 (21.228)
Median	75.00	8.33	66.67	4.17	66.67	4.17
Min, Max	58.3, 100.0	-16.7, 33.3	16.7, 83.3	-33.3, 41.7	16.7, 100.0	-33.3, 41.7

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Global Health Status/QoL

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	86.11 (24.056)	11.11 (19.245)	79.17 (5.893)	25.00 (23.570)	83.33 (17.678)	16.67 (19.543)
Median	100.00	0.00	79.17	25.00	83.33	8.33
Min, Max	58.3, 100.0	0.0, 33.3	75.0, 83.3	8.3, 41.7	58.3, 100.0	0.0, 41.7

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Modified Randomized Set

Physical Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		15		49	
Mean (SD)	71.96 (18.480)		60.00 (22.396)		68.30 (20.300)	
Median	76.67		66.67		66.67	
Min, Max	40.0, 100.0		26.7, 93.3		26.7, 100.0	
Week 2						
n	34	34	17	15		
Mean (SD)	75.88 (17.292)	3.92 (10.395)	61.96 (18.821)	1.33 (14.075)		
Median	80.00	0.00	66.67	0.00		
Min, Max	40.0, 100.0	-20.0, 26.7	26.7, 93.3	-13.3, 40.0		

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Physical Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	34	34	16	15		
Mean (SD)	81.76 (16.762)	9.80 (14.141)	62.50 (21.892)	0.89 (20.910)		
Median	86.67	6.67	60.00	0.00		
Min, Max	40.0, 100.0	-20.0, 46.7	26.7, 93.3	-26.7, 53.3		
Week 8						
n	26	26	12	11		
Mean (SD)	82.05 (16.521)	11.54 (16.499)	65.00 (22.940)	4.24 (21.761)		
Median	83.33	13.33	70.00	0.00		
Min, Max	33.3, 100.0	-26.7, 46.7	20.0, 100.0	-26.7, 46.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Physical Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	16	15		
Mean (SD)	84.22 (13.503)	11.33 (13.913)	62.50 (21.344)	1.33 (19.873)		
Median	86.67	6.67	70.00	0.00		
Min, Max	53.3, 100.0	-6.7, 46.7	20.0, 93.3	-33.3, 46.7		
Week 24						
n	28	28	15	14		
Mean (SD)	84.29 (13.604)	10.71 (17.901)	76.00 (23.745)	12.86 (13.452)		
Median	86.67	10.00	86.67	13.33		
Min, Max	53.3, 100.0	-33.3, 53.3	26.7, 100.0	-6.7, 40.0		

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Table GERMANY.2.5.11.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Physical Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	25	25	13	13	38	38
Mean (SD)	82.67 (14.272)	8.27 (14.179)	69.74 (26.051)	4.62 (15.726)	78.25 (19.769)	7.02 (14.618)
Median	86.67	6.67	73.33	0.00	83.33	6.67
Min, Max	60.0, 100.0	-26.7, 46.7	20.0, 100.0	-26.7, 26.7	20.0, 100.0	-26.7, 46.7
Week 56						
n	17	17	12	11	29	28
Mean (SD)	80.39 (15.179)	5.88 (12.885)	76.67 (14.634)	7.27 (15.904)	78.85 (14.808)	6.43 (13.876)
Median	80.00	6.67	80.00	13.33	80.00	6.67
Min, Max	53.3, 100.0	-20.0, 26.7	53.3, 100.0	-20.0, 33.3	53.3, 100.0	-20.0, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Physical Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	10	9	23	22
Mean (SD)	82.56 (14.792)	5.64 (14.869)	72.67 (26.378)	3.70 (19.181)	78.26 (20.716)	4.85 (16.353)
Median	86.67	6.67	80.00	6.67	86.67	6.67
Min, Max	53.3, 100.0	-13.3, 26.7	6.7, 93.3	-40.0, 20.0	6.7, 100.0	-40.0, 26.7
Week 88						
n	8	8	6	6	14	14
Mean (SD)	88.33 (12.215)	9.17 (10.653)	73.33 (24.944)	7.78 (18.579)	81.90 (19.468)	8.57 (13.942)
Median	90.00	6.67	76.67	3.33	86.67	6.67
Min, Max	66.7, 100.0	-6.7, 26.7	33.3, 100.0	-13.3, 33.3	33.3, 100.0	-13.3, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Physical Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	88.89 (13.878)	8.89 (7.698)	90.00 (4.714)	20.00 (18.856)	89.33 (10.111)	13.33 (12.472)
Median	93.33	13.33	90.00	20.00	93.33	13.33
Min, Max	73.3, 100.0	0.0, 13.3	86.7, 93.3	6.7, 33.3	73.3, 100.0	0.0, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Role Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		15		49	
Mean (SD)	64.71 (26.832)		57.78 (30.775)		62.59 (27.957)	
Median	66.67		66.67		66.67	
Min, Max	0.0, 100.0		16.7, 100.0		0.0, 100.0	
Week 2						
n	33	33	16	14		
Mean (SD)	74.24 (28.895)	9.60 (20.846)	64.58 (31.549)	4.76 (17.817)		
Median	83.33	0.00	66.67	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Role Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	34	34	15	14		
Mean (SD)	80.39 (23.018)	15.69 (26.572)	63.33 (29.681)	1.19 (32.334)		
Median	83.33	16.67	66.67	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	16.7, 100.0	-33.3, 83.3		
Week 8						
n	26	26	11	10		
Mean (SD)	81.41 (19.623)	19.87 (23.103)	65.15 (32.020)	1.67 (35.530)		
Median	83.33	16.67	66.67	0.00		
Min, Max	33.3, 100.0	-16.7, 83.3	16.7, 100.0	-66.7, 66.7		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
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Role Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	15	14		
Mean (SD)	82.78 (18.817)	17.22 (25.327)	64.44 (30.123)	4.76 (37.796)		
Median	83.33	16.67	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 66.7	0.0, 100.0	-66.7, 66.7		
Week 24						
n	28	28	15	14		
Mean (SD)	75.00 (29.222)	8.93 (29.911)	70.00 (35.187)	10.71 (43.661)		
Median	83.33	8.33	83.33	25.00		
Min, Max	0.0, 100.0	-66.7, 50.0	0.0, 100.0	-100.0, 66.7		

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Role Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	25	25	13	13	38	38
Mean (SD)	81.33 (21.148)	12.67 (26.034)	62.82 (37.978)	3.85 (29.778)	75.00 (28.932)	9.65 (27.299)
Median	83.33	0.00	66.67	0.00	66.67	0.00
Min, Max	16.7, 100.0	-50.0, 66.7	0.0, 100.0	-50.0, 50.0	0.0, 100.0	-50.0, 66.7
Week 56						
n	17	17	12	11	29	28
Mean (SD)	72.55 (22.002)	0.00 (22.822)	77.78 (14.794)	13.64 (31.463)	74.71 (19.221)	5.36 (26.856)
Median	66.67	0.00	66.67	16.67	66.67	0.00
Min, Max	33.3, 100.0	-33.3, 50.0	66.7, 100.0	-33.3, 66.7	33.3, 100.0	-33.3, 66.7

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Role Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	9	8	22	21
Mean (SD)	78.21 (32.903)	7.69 (22.169)	74.07 (22.222)	14.58 (22.603)	76.52 (28.480)	10.32 (22.033)
Median	100.00	0.00	66.67	25.00	91.67	0.00
Min, Max	0.0, 100.0	-33.3, 50.0	50.0, 100.0	-16.7, 33.3	0.0, 100.0	-33.3, 50.0
Week 88						
n	8	8	6	6	14	14
Mean (SD)	81.25 (27.368)	4.17 (14.773)	66.67 (29.814)	5.56 (31.032)	75.00 (28.307)	4.76 (22.100)
Median	100.00	0.00	75.00	0.00	83.33	0.00
Min, Max	33.3, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 66.7	16.7, 100.0	-16.7, 66.7

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Role Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	83.33 (28.868)	5.56 (9.623)	83.33 (23.570)	33.33 (23.570)	83.33 (23.570)	16.67 (20.412)
Median	100.00	0.00	83.33	33.33	100.00	16.67
Min, Max	50.0, 100.0	0.0, 16.7	66.7, 100.0	16.7, 50.0	50.0, 100.0	0.0, 50.0

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Table GERMANY.2.5.11.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Emotional Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		15		49	
Mean (SD)	79.17 (21.443)		66.11 (27.180)		75.17 (23.844)	
Median	83.33		66.67		75.00	
Min, Max	0.0, 100.0		8.3, 100.0		0.0, 100.0	
Week 2						
n	33	33	17	15		
Mean (SD)	84.09 (18.442)	5.05 (16.528)	69.12 (23.527)	3.33 (16.306)		
Median	91.67	0.00	75.00	0.00		
Min, Max	41.7, 100.0	-25.0, 58.3	33.3, 100.0	-33.3, 25.0		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Emotional Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	34	34	16	15		
Mean (SD)	82.11 (25.716)	2.94 (21.893)	68.23 (22.406)	0.00 (18.366)		
Median	91.67	0.00	66.67	0.00		
Min, Max	0.0, 100.0	-66.7, 58.3	25.0, 100.0	-33.3, 33.3		
Week 8						
n	26	26	12	11		
Mean (SD)	84.94 (19.151)	8.65 (21.145)	77.08 (23.335)	5.30 (11.348)		
Median	91.67	8.33	83.33	8.33		
Min, Max	41.7, 100.0	-25.0, 58.3	16.7, 100.0	-16.7, 16.7		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Emotional Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	16	15		
Mean (SD)	88.61 (16.878)	9.72 (15.945)	66.67 (23.766)	0.00 (17.252)		
Median	100.00	8.33	70.83	0.00		
Min, Max	41.7, 100.0	-8.3, 66.7	0.0, 100.0	-50.0, 25.0		
Week 24						
n	28	28	15	14		
Mean (SD)	82.74 (22.444)	5.36 (23.042)	74.44 (18.758)	8.33 (19.882)		
Median	91.67	0.00	75.00	8.33		
Min, Max	0.0, 100.0	-66.7, 66.7	33.3, 100.0	-33.3, 50.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Emotional Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	25	25	13	13	38	38
Mean (SD)	83.67 (23.751)	3.00 (23.432)	72.44 (18.125)	7.05 (22.269)	79.82 (22.397)	4.39 (22.821)
Median	91.67	0.00	75.00	0.00	87.50	0.00
Min, Max	0.0, 100.0	-66.7, 58.3	25.0, 100.0	-25.0, 66.7	0.0, 100.0	-66.7, 66.7
Week 56						
n	17	17	12	11	29	28
Mean (SD)	78.92 (27.022)	0.98 (26.333)	74.31 (17.929)	9.85 (28.338)	77.01 (23.428)	4.46 (26.978)
Median	91.67	0.00	79.17	0.00	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 50.0	41.7, 100.0	-16.7, 75.0	0.0, 100.0	-66.7, 75.0

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Emotional Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	10	9	22	21
Mean (SD)	90.28 (15.006)	2.08 (15.941)	71.67 (23.960)	5.56 (16.667)	81.82 (21.306)	3.57 (15.936)
Median	100.00	0.00	75.00	8.33	91.67	0.00
Min, Max	58.3, 100.0	-33.3, 25.0	25.0, 100.0	-25.0, 33.3	25.0, 100.0	-33.3, 33.3
Week 88						
n	8	8	6	6	14	14
Mean (SD)	89.58 (14.605)	5.21 (10.854)	69.44 (23.960)	13.89 (41.051)	80.95 (21.038)	8.93 (27.045)
Median	95.83	0.00	75.00	12.50	91.67	0.00
Min, Max	66.7, 100.0	-8.3, 25.0	33.3, 91.7	-33.3, 83.3	33.3, 100.0	-33.3, 83.3

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Table GERMANY.2.5.11.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Emotional Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	94.44 (9.623)	2.78 (4.811)	62.50 (41.248)	25.00 (82.496)	81.67 (27.889)	11.67 (43.141)
Median	100.00	0.00	62.50	25.00	91.67	0.00
Min, Max	83.3, 100.0	0.0, 8.3	33.3, 91.7	-33.3, 83.3	33.3, 100.0	-33.3, 83.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Cognitive Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		15		49	
Mean (SD)	79.90 (21.626)		60.00 (27.314)		73.81 (25.000)	
Median	83.33		66.67		66.67	
Min, Max	16.7, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	33	33	16	15		
Mean (SD)	83.33 (19.094)	3.54 (16.540)	66.67 (30.429)	5.56 (26.478)		
Median	83.33	0.00	75.00	0.00		
Min, Max	33.3, 100.0	-16.7, 50.0	0.0, 100.0	-33.3, 66.7		

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Cognitive Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	34	34	16	15		
Mean (SD)	84.31 (18.322)	4.41 (20.642)	61.46 (32.041)	0.00 (25.198)		
Median	83.33	0.00	66.67	0.00		
Min, Max	16.7, 100.0	-50.0, 50.0	0.0, 100.0	-33.3, 50.0		
Week 8						
n	26	26	12	11		
Mean (SD)	85.26 (17.209)	9.62 (20.096)	69.44 (30.011)	12.12 (21.201)		
Median	83.33	0.00	75.00	0.00		
Min, Max	33.3, 100.0	-33.3, 66.7	0.0, 100.0	-16.7, 50.0		

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Cognitive Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	16	15		
Mean (SD)	86.67 (16.022)	7.22 (15.588)	68.75 (29.107)	7.78 (24.289)		
Median	100.00	0.00	75.00	0.00		
Min, Max	50.0, 100.0	-16.7, 33.3	16.7, 100.0	-33.3, 50.0		
Week 24						
n	28	28	15	14		
Mean (SD)	85.71 (21.619)	7.14 (22.420)	72.22 (24.125)	10.71 (16.803)		
Median	100.00	0.00	83.33	16.67		
Min, Max	0.0, 100.0	-66.7, 50.0	33.3, 100.0	-16.7, 33.3		

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Cognitive Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	25	25	13	13	38	38
Mean (SD)	84.67 (23.034)	5.33 (22.933)	62.82 (30.546)	-1.28 (18.586)	77.19 (27.515)	3.07 (21.524)
Median	100.00	0.00	66.67	0.00	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 50.0	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 50.0
Week 56						
n	17	17	12	11	29	28
Mean (SD)	88.24 (21.053)	2.94 (21.437)	68.06 (35.146)	0.00 (22.361)	79.89 (28.998)	1.79 (21.440)
Median	100.00	0.00	83.33	0.00	83.33	0.00
Min, Max	16.7, 100.0	-50.0, 33.3	0.0, 100.0	-50.0, 33.3	0.0, 100.0	-50.0, 33.3

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Table GERMANY.2.5.11.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Cognitive Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	9	8	21	20
Mean (SD)	94.44 (10.856)	5.56 (14.794)	68.52 (31.672)	4.17 (14.773)	83.33 (25.276)	5.00 (14.408)
Median	100.00	0.00	83.33	8.33	100.00	0.00
Min, Max	66.7, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 16.7	16.7, 100.0	-16.7, 33.3
Week 88						
n	8	8	6	6	14	14
Mean (SD)	91.67 (12.599)	6.25 (12.400)	50.00 (39.441)	-2.78 (26.701)	73.81 (33.788)	2.38 (19.455)
Median	100.00	0.00	50.00	0.00	83.33	0.00
Min, Max	66.7, 100.0	0.0, 33.3	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 33.3

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Table GERMANY.2.5.11.2
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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Cognitive Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	88.89 (19.245)	0.00 (0.000)	58.33 (35.355)	-8.33 (35.355)	76.67 (27.889)	-3.33 (18.257)
Median	100.00	0.00	58.33	-8.33	83.33	0.00
Min, Max	66.7, 100.0	0.0, 0.0	33.3, 83.3	-33.3, 16.7	33.3, 100.0	-33.3, 16.7

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Table GERMANY.2.5.11.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Social Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		15		49	
Mean (SD)	72.06 (21.989)		63.33 (36.297)		69.39 (27.077)	
Median	66.67		83.33		66.67	
Min, Max	16.7, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	34	34	16	14		
Mean (SD)	80.88 (22.146)	8.82 (15.481)	66.67 (32.203)	1.19 (22.134)		
Median	83.33	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-16.7, 50.0	0.0, 100.0	-33.3, 50.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Social Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	34	34	15	14		
Mean (SD)	79.90 (23.130)	7.84 (17.995)	64.44 (28.776)	1.19 (26.525)		
Median	83.33	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 50.0	0.0, 100.0	-50.0, 50.0		
Week 8						
n	26	26	11	10		
Mean (SD)	87.18 (18.444)	16.67 (22.608)	80.30 (26.686)	8.33 (18.002)		
Median	100.00	16.67	100.00	8.33		
Min, Max	33.3, 100.0	-16.7, 83.3	16.7, 100.0	-16.7, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Social Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	16	15		
Mean (SD)	86.11 (17.553)	13.89 (19.120)	59.38 (34.944)	-6.67 (30.079)		
Median	100.00	8.33	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 50.0	0.0, 100.0	-83.3, 33.3		
Week 24						
n	28	28	14	13		
Mean (SD)	78.57 (26.001)	5.36 (25.278)	71.43 (30.261)	11.54 (25.806)		
Median	91.67	0.00	75.00	16.67		
Min, Max	0.0, 100.0	-66.7, 50.0	0.0, 100.0	-33.3, 50.0		

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Social Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	25	25	13	13	38	38
Mean (SD)	76.00 (27.251)	2.00 (24.210)	57.69 (38.258)	-5.13 (45.330)	69.74 (32.154)	-0.44 (32.532)
Median	83.33	0.00	66.67	0.00	66.67	0.00
Min, Max	0.0, 100.0	-66.7, 50.0	0.0, 100.0	-100.0, 50.0	0.0, 100.0	-100.0, 50.0
Week 56						
n	17	17	12	11	29	28
Mean (SD)	81.37 (20.312)	6.86 (17.735)	75.00 (21.904)	9.09 (32.799)	78.74 (20.844)	7.74 (24.209)
Median	83.33	0.00	66.67	0.00	66.67	0.00
Min, Max	33.3, 100.0	-33.3, 33.3	33.3, 100.0	-50.0, 66.7	33.3, 100.0	-50.0, 66.7

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Table GERMANY.2.5.11.2
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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Social Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	9	8	21	20
Mean (SD)	86.11 (21.122)	11.11 (21.711)	75.93 (26.498)	-2.08 (35.003)	81.75 (23.514)	5.83 (27.718)
Median	100.00	0.00	83.33	16.67	83.33	8.33
Min, Max	33.3, 100.0	-16.7, 50.0	16.7, 100.0	-83.3, 16.7	16.7, 100.0	-83.3, 50.0
Week 88						
n	8	8	6	6	14	14
Mean (SD)	81.25 (16.517)	4.17 (14.773)	77.78 (17.213)	13.89 (32.347)	79.76 (16.249)	8.33 (23.342)
Median	75.00	0.00	66.67	16.67	66.67	0.00
Min, Max	66.7, 100.0	-16.7, 33.3	66.7, 100.0	-33.3, 66.7	66.7, 100.0	-33.3, 66.7

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Table GERMANY.2.5.11.2
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Social Functioning

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	88.89 (19.245)	0.00 (33.333)	83.33 (23.570)	58.33 (11.785)	86.67 (18.257)	23.33 (40.139)
Median	100.00	0.00	83.33	58.33	100.00	33.33
Min, Max	66.7, 100.0	-33.3, 33.3	66.7, 100.0	50.0, 66.7	66.7, 100.0	-33.3, 66.7

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Global Health Status/QoL

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	41		18		59	
Mean (SD)	56.50 (21.851)		54.63 (18.573)		55.93 (20.764)	
Median	66.67		58.33		58.33	
Min, Max	16.7, 100.0		16.7, 91.7		16.7, 100.0	
Week 2						
n	41	41	20	18		
Mean (SD)	63.62 (21.308)	7.11 (17.927)	57.50 (15.972)	2.31 (18.481)		
Median	66.67	8.33	58.33	0.00		
Min, Max	16.7, 100.0	-50.0, 41.7	25.0, 83.3	-25.0, 50.0		

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Global Health Status/QoL

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	41	41	19	18		
Mean (SD)	65.85 (21.874)	9.35 (16.478)	55.70 (24.383)	-0.46 (18.184)		
Median	66.67	0.00	50.00	0.00		
Min, Max	25.0, 100.0	-16.7, 50.0	25.0, 100.0	-41.7, 41.7		
Week 8						
n	32	32	14	13		
Mean (SD)	67.19 (22.889)	10.42 (22.401)	60.12 (23.154)	2.56 (20.801)		
Median	66.67	12.50	66.67	0.00		
Min, Max	16.7, 100.0	-58.3, 50.0	16.7, 83.3	-33.3, 41.7		

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Global Health Status/QoL

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	39	39	19	18		
Mean (SD)	69.66 (19.913)	13.46 (14.761)	62.28 (17.649)	6.94 (17.678)		
Median	66.67	16.67	66.67	8.33		
Min, Max	33.3, 100.0	-16.7, 41.7	33.3, 91.7	-25.0, 33.3		
Week 24						
n	36	36	18	17		
Mean (SD)	69.68 (20.134)	12.04 (15.742)	68.06 (20.262)	12.75 (14.166)		
Median	66.67	16.67	66.67	8.33		
Min, Max	33.3, 100.0	-33.3, 41.7	16.7, 100.0	-8.3, 41.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Global Health Status/QoL

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	33	33	16	16	49	49
Mean (SD)	69.19 (21.091)	11.62 (16.129)	63.54 (25.797)	5.73 (17.930)	67.35 (22.620)	9.69 (16.783)
Median	66.67	16.67	66.67	8.33	66.67	8.33
Min, Max	33.3, 100.0	-41.7, 33.3	16.7, 100.0	-33.3, 33.3	16.7, 100.0	-41.7, 33.3
Week 56						
n	23	23	15	14	38	37
Mean (SD)	68.48 (19.292)	10.14 (15.882)	62.78 (21.563)	2.38 (21.787)	66.23 (20.130)	7.21 (18.443)
Median	66.67	8.33	66.67	0.00	66.67	0.00
Min, Max	33.3, 100.0	-16.7, 41.7	8.3, 83.3	-25.0, 41.7	8.3, 100.0	-25.0, 41.7

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Global Health Status/QoL

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	14	14	10	9	24	23
Mean (SD)	68.45 (18.251)	10.71 (25.409)	66.67 (22.567)	6.48 (25.947)	67.71 (19.707)	9.06 (25.115)
Median	66.67	16.67	70.83	8.33	66.67	16.67
Min, Max	33.3, 100.0	-50.0, 41.7	16.7, 91.7	-33.3, 50.0	16.7, 100.0	-50.0, 50.0
Week 88						
n	8	8	6	6	14	14
Mean (SD)	79.17 (17.817)	16.67 (9.960)	70.83 (12.638)	13.89 (18.002)	75.60 (15.833)	15.48 (13.420)
Median	83.33	16.67	70.83	12.50	83.33	16.67
Min, Max	41.7, 100.0	0.0, 33.3	50.0, 83.3	-8.3, 41.7	41.7, 100.0	-8.3, 41.7

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Table GERMANY.2.5.11.3

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Global Health Status/QoL

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	88.89 (19.245)	11.11 (19.245)	79.17 (5.893)	25.00 (23.570)	85.00 (14.907)	16.67 (19.543)
Median	100.00	0.00	79.17	25.00	83.33	8.33
Min, Max	66.7, 100.0	0.0, 33.3	75.0, 83.3	8.3, 41.7	66.7, 100.0	0.0, 41.7

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Physical Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	41		18		59	
Mean (SD)	72.68 (20.265)		64.81 (22.265)		70.28 (21.021)	
Median	80.00		66.67		73.33	
Min, Max	33.3, 100.0		26.7, 100.0		26.7, 100.0	
Week 2						
n	40	40	20	18		
Mean (SD)	80.17 (18.272)	8.00 (12.002)	66.33 (19.161)	1.48 (13.442)		
Median	86.67	0.00	70.00	0.00		
Min, Max	26.7, 100.0	-6.7, 46.7	26.7, 100.0	-13.3, 40.0		

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Modified Randomized Set

Physical Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	41	41	19	18		
Mean (SD)	84.23 (16.381)	11.54 (14.834)	67.37 (22.321)	1.48 (19.611)		
Median	86.67	13.33	66.67	3.33		
Min, Max	40.0, 100.0	-20.0, 60.0	26.7, 100.0	-26.7, 53.3		
Week 8						
n	32	32	14	13		
Mean (SD)	87.08 (13.540)	12.71 (14.204)	72.38 (18.277)	7.18 (17.943)		
Median	90.00	13.33	73.33	0.00		
Min, Max	53.3, 100.0	-13.3, 46.7	33.3, 100.0	-20.0, 46.7		

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Modified Randomized Set

Physical Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	39	39	19	18		
Mean (SD)	86.32 (16.182)	12.48 (13.865)	67.72 (19.533)	2.22 (18.994)		
Median	86.67	13.33	73.33	3.33		
Min, Max	26.7, 100.0	-6.7, 46.7	33.3, 100.0	-33.3, 46.7		
Week 24						
n	36	36	18	17		
Mean (SD)	87.96 (14.590)	13.33 (17.530)	81.48 (22.235)	14.12 (12.885)		
Median	93.33	13.33	86.67	13.33		
Min, Max	40.0, 100.0	-33.3, 60.0	26.7, 100.0	-6.7, 40.0		

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Physical Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	33	33	16	16	49	49
Mean (SD)	85.25 (18.391)	10.30 (15.907)	77.92 (21.183)	8.33 (12.049)	82.86 (19.437)	9.66 (14.660)
Median	86.67	13.33	80.00	6.67	86.67	6.67
Min, Max	13.3, 100.0	-26.7, 53.3	33.3, 100.0	-6.7, 26.7	13.3, 100.0	-26.7, 53.3
Week 56						
n	23	23	15	14	38	37
Mean (SD)	86.38 (16.015)	12.17 (14.723)	80.44 (15.830)	8.10 (14.601)	84.04 (15.998)	10.63 (14.611)
Median	93.33	13.33	80.00	13.33	86.67	13.33
Min, Max	46.7, 100.0	-13.3, 60.0	53.3, 100.0	-20.0, 33.3	46.7, 100.0	-20.0, 60.0

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Modified Randomized Set

Physical Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	15	15	10	9	25	24
Mean (SD)	86.67 (13.093)	12.89 (18.077)	82.00 (14.072)	10.37 (10.599)	84.80 (13.405)	11.94 (15.477)
Median	93.33	13.33	86.67	13.33	86.67	13.33
Min, Max	60.0, 100.0	-13.3, 60.0	60.0, 100.0	-6.7, 20.0	60.0, 100.0	-13.3, 60.0
Week 88						
n	8	8	6	6	14	14
Mean (SD)	89.17 (14.226)	10.83 (10.040)	84.44 (17.213)	13.33 (15.776)	87.14 (15.127)	11.90 (12.315)
Median	93.33	6.67	90.00	13.33	90.00	6.67
Min, Max	60.0, 100.0	0.0, 26.7	60.0, 100.0	-6.7, 33.3	60.0, 100.0	-6.7, 33.3

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Physical Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	97.78 (3.849)	6.67 (6.667)	90.00 (4.714)	20.00 (18.856)	94.67 (5.578)	12.00 (12.824)
Median	100.00	6.67	90.00	20.00	93.33	6.67
Min, Max	93.3, 100.0	0.0, 13.3	86.7, 93.3	6.7, 33.3	86.7, 100.0	0.0, 33.3

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Role Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	41		18		59	
Mean (SD)	64.23 (29.002)		61.11 (28.006)		63.28 (28.497)	
Median	66.67		66.67		66.67	
Min, Max	0.0, 100.0		16.7, 100.0		0.0, 100.0	
Week 2						
n	40	40	20	18		
Mean (SD)	75.00 (30.194)	11.67 (24.226)	65.00 (28.562)	3.70 (16.721)		
Median	83.33	0.00	66.67	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 33.3		

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Role Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	41	41	19	18		
Mean (SD)	80.08 (25.879)	15.85 (28.856)	65.79 (28.040)	2.78 (28.726)		
Median	83.33	16.67	66.67	0.00		
Min, Max	0.0, 100.0	-66.7, 83.3	16.7, 100.0	-33.3, 83.3		
Week 8						
n	32	32	14	13		
Mean (SD)	82.81 (20.947)	19.27 (23.612)	66.67 (28.495)	1.28 (31.521)		
Median	83.33	16.67	66.67	0.00		
Min, Max	33.3, 100.0	-16.7, 83.3	16.7, 100.0	-66.7, 66.7		

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Role Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	39	39	19	18		
Mean (SD)	82.05 (23.996)	17.09 (23.721)	67.54 (32.142)	6.48 (35.304)		
Median	100.00	16.67	83.33	0.00		
Min, Max	0.0, 100.0	-16.7, 66.7	0.0, 100.0	-66.7, 66.7		
Week 24						
n	36	36	18	17		
Mean (SD)	79.63 (27.635)	14.35 (29.855)	74.07 (33.442)	11.76 (38.982)		
Median	91.67	0.00	83.33	16.67		
Min, Max	0.0, 100.0	-66.7, 83.3	0.0, 100.0	-100.0, 66.7		

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Modified Randomized Set

Role Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	33	33	16	16	49	49
Mean (SD)	81.82 (24.778)	14.65 (26.272)	73.96 (31.604)	11.46 (26.330)	79.25 (27.117)	13.61 (26.059)
Median	100.00	0.00	83.33	16.67	100.00	0.00
Min, Max	0.0, 100.0	-50.0, 66.7	0.0, 100.0	-50.0, 50.0	0.0, 100.0	-50.0, 66.7
Week 56						
n	23	23	15	14	38	37
Mean (SD)	76.09 (24.529)	8.70 (25.559)	81.11 (15.258)	16.67 (28.495)	78.07 (21.261)	11.71 (26.604)
Median	66.67	0.00	83.33	16.67	75.00	0.00
Min, Max	16.7, 100.0	-33.3, 66.7	66.7, 100.0	-33.3, 66.7	16.7, 100.0	-33.3, 66.7

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Modified Randomized Set

Role Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	15	15	10	9	25	24
Mean (SD)	83.33 (30.211)	20.00 (28.312)	75.00 (21.155)	14.81 (21.155)	80.00 (26.788)	18.06 (25.498)
Median	100.00	16.67	75.00	16.67	100.00	16.67
Min, Max	0.0, 100.0	-33.3, 66.7	50.0, 100.0	-16.7, 33.3	0.0, 100.0	-33.3, 66.7
Week 88						
n	8	8	6	6	14	14
Mean (SD)	87.50 (24.801)	14.58 (22.603)	80.56 (19.484)	13.89 (30.581)	84.52 (22.134)	14.29 (25.198)
Median	100.00	8.33	83.33	0.00	100.00	0.00
Min, Max	33.3, 100.0	-16.7, 50.0	50.0, 100.0	-16.7, 66.7	33.3, 100.0	-16.7, 66.7

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Role Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	100.00 (0.000)	0.00 (0.000)	83.33 (23.570)	33.33 (23.570)	93.33 (14.907)	13.33 (21.731)
Median	100.00	0.00	83.33	33.33	100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0	66.7, 100.0	16.7, 50.0	66.7, 100.0	0.0, 50.0

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Modified Randomized Set

Emotional Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	41		18		59	
Mean (SD)	78.66 (25.280)		68.52 (25.494)		75.56 (25.562)	
Median	83.33		70.83		83.33	
Min, Max	0.0, 100.0		8.3, 100.0		0.0, 100.0	
Week 2						
n	41	41	20	18		
Mean (SD)	85.57 (19.187)	6.91 (16.760)	72.08 (22.826)	4.17 (14.921)		
Median	100.00	0.00	79.17	0.00		
Min, Max	33.3, 100.0	-25.0, 58.3	33.3, 100.0	-33.3, 25.0		

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Modified Randomized Set

Emotional Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	41	41	19	18		
Mean (SD)	82.32 (25.083)	3.66 (21.250)	75.44 (23.972)	5.56 (16.169)		
Median	91.67	0.00	83.33	0.00		
Min, Max	0.0, 100.0	-66.7, 58.3	25.0, 100.0	-33.3, 33.3		
Week 8						
n	32	32	14	13		
Mean (SD)	85.94 (16.591)	8.07 (18.390)	83.93 (23.675)	11.54 (9.941)		
Median	91.67	8.33	95.83	16.67		
Min, Max	41.7, 100.0	-25.0, 58.3	16.7, 100.0	0.0, 33.3		

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Modified Randomized Set

Emotional Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	39	39	19	18		
Mean (SD)	86.75 (20.207)	8.12 (14.621)	73.25 (25.395)	4.63 (14.913)		
Median	100.00	0.00	75.00	4.17		
Min, Max	16.7, 100.0	-8.3, 66.7	0.0, 100.0	-33.3, 33.3		
Week 24						
n	36	36	18	17		
Mean (SD)	83.56 (24.840)	5.56 (20.509)	79.17 (19.227)	10.78 (19.267)		
Median	91.67	0.00	83.33	8.33		
Min, Max	0.0, 100.0	-66.7, 66.7	33.3, 100.0	-33.3, 50.0		

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Emotional Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	33	33	16	16	49	49
Mean (SD)	84.09 (24.770)	4.29 (20.637)	79.69 (20.177)	11.46 (19.215)	82.65 (23.251)	6.63 (20.269)
Median	100.00	8.33	75.00	8.33	91.67	8.33
Min, Max	0.0, 100.0	-66.7, 58.3	25.0, 100.0	-8.3, 66.7	0.0, 100.0	-66.7, 66.7
Week 56						
n	23	23	15	14	38	37
Mean (SD)	80.80 (26.642)	3.26 (24.197)	77.78 (18.545)	8.93 (27.632)	79.61 (23.546)	5.41 (25.323)
Median	100.00	0.00	83.33	0.00	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 50.0	41.7, 100.0	-16.7, 75.0	0.0, 100.0	-66.7, 75.0

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Emotional Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	14	14	10	9	24	23
Mean (SD)	91.07 (12.431)	3.57 (15.578)	74.17 (25.595)	12.04 (14.500)	84.03 (20.400)	6.88 (15.418)
Median	100.00	0.00	75.00	8.33	91.67	0.00
Min, Max	66.7, 100.0	-33.3, 33.3	25.0, 100.0	-8.3, 33.3	25.0, 100.0	-33.3, 33.3
Week 88						
n	8	8	6	6	14	14
Mean (SD)	93.75 (11.573)	6.25 (11.573)	75.00 (26.874)	25.00 (34.157)	85.71 (21.038)	14.29 (24.770)
Median	100.00	0.00	87.50	25.00	91.67	8.33
Min, Max	66.7, 100.0	-8.3, 25.0	33.3, 100.0	-16.7, 83.3	33.3, 100.0	-16.7, 83.3

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Emotional Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	100.00 (0.000)	0.00 (0.000)	62.50 (41.248)	25.00 (82.496)	85.00 (29.107)	10.00 (43.461)
Median	100.00	0.00	62.50	25.00	100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0	33.3, 91.7	-33.3, 83.3	33.3, 100.0	-33.3, 83.3

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Cognitive Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	41		18		59	
Mean (SD)	77.24 (24.369)		67.59 (27.100)		74.29 (25.394)	
Median	83.33		66.67		83.33	
Min, Max	16.7, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	41	41	19	18		
Mean (SD)	84.55 (18.024)	7.32 (18.276)	76.32 (25.041)	8.33 (23.743)		
Median	83.33	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-16.7, 66.7	16.7, 100.0	-33.3, 66.7		

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Cognitive Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	41	41	19	18		
Mean (SD)	84.55 (19.145)	7.32 (20.086)	73.68 (29.036)	5.56 (24.254)		
Median	83.33	0.00	83.33	0.00		
Min, Max	16.7, 100.0	-50.0, 50.0	0.0, 100.0	-33.3, 50.0		
Week 8						
n	32	32	14	13		
Mean (SD)	85.94 (14.731)	11.46 (17.163)	79.76 (27.872)	14.10 (20.237)		
Median	83.33	8.33	83.33	16.67		
Min, Max	50.0, 100.0	-16.7, 66.7	0.0, 100.0	-16.7, 50.0		

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Cognitive Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	39	39	19	18		
Mean (SD)	86.32 (17.045)	9.40 (14.707)	77.19 (25.586)	9.26 (22.304)		
Median	100.00	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-16.7, 50.0	16.7, 100.0	-33.3, 50.0		
Week 24						
n	36	36	18	17		
Mean (SD)	83.33 (24.560)	6.94 (20.845)	79.63 (22.547)	10.78 (16.605)		
Median	91.67	0.00	83.33	16.67		
Min, Max	0.0, 100.0	-66.7, 50.0	33.3, 100.0	-16.7, 33.3		

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Cognitive Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	33	33	16	16	49	49
Mean (SD)	82.83 (23.005)	7.58 (20.451)	75.00 (25.092)	3.13 (16.351)	80.27 (23.735)	6.12 (19.153)
Median	83.33	0.00	83.33	0.00	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 50.0	16.7, 100.0	-16.7, 33.3	0.0, 100.0	-66.7, 50.0
Week 56						
n	23	23	15	14	38	37
Mean (SD)	86.23 (22.837)	7.97 (20.019)	72.22 (33.134)	1.19 (22.134)	80.70 (27.815)	5.41 (20.807)
Median	100.00	0.00	83.33	0.00	91.67	0.00
Min, Max	16.7, 100.0	-50.0, 50.0	0.0, 100.0	-50.0, 33.3	0.0, 100.0	-50.0, 50.0

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Modified Randomized Set

Cognitive Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	14	14	9	8	23	22
Mean (SD)	90.48 (14.194)	7.14 (16.939)	77.78 (26.352)	10.42 (15.269)	85.51 (20.291)	8.33 (16.060)
Median	100.00	0.00	83.33	16.67	100.00	0.00
Min, Max	66.7, 100.0	-16.7, 50.0	16.7, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 50.0
Week 88						
n	8	8	6	6	14	14
Mean (SD)	93.75 (12.400)	8.33 (15.430)	66.67 (34.960)	8.33 (25.276)	82.14 (27.319)	8.33 (19.337)
Median	100.00	0.00	75.00	8.33	100.00	0.00
Min, Max	66.7, 100.0	0.0, 33.3	16.7, 100.0	-33.3, 33.3	16.7, 100.0	-33.3, 33.3

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Cognitive Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	100.00 (0.000)	0.00 (0.000)	58.33 (35.355)	-8.33 (35.355)	83.33 (28.868)	-3.33 (18.257)
Median	100.00	0.00	58.33	-8.33	100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0	33.3, 83.3	-33.3, 16.7	33.3, 100.0	-33.3, 16.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Social Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	41		18		59	
Mean (SD)	71.95 (27.998)		60.19 (30.859)		68.36 (29.147)	
Median	66.67		66.67		66.67	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	41	41	20	18		
Mean (SD)	80.49 (22.330)	8.54 (25.310)	67.50 (28.855)	3.70 (21.047)		
Median	83.33	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-50.0, 83.3	0.0, 100.0	-33.3, 50.0		

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Social Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	41	41	19	18		
Mean (SD)	80.49 (22.017)	8.54 (24.187)	66.67 (27.778)	4.63 (24.790)		
Median	83.33	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 83.3	0.0, 100.0	-50.0, 50.0		
Week 8						
n	32	32	14	13		
Mean (SD)	86.98 (19.277)	13.54 (24.479)	77.38 (24.114)	8.97 (18.777)		
Median	100.00	8.33	66.67	16.67		
Min, Max	33.3, 100.0	-50.0, 83.3	16.7, 100.0	-16.7, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Social Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	39	39	19	18		
Mean (SD)	84.62 (20.727)	12.39 (22.852)	64.04 (31.063)	1.85 (22.787)		
Median	100.00	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 83.3	0.0, 100.0	-66.7, 33.3		
Week 24						
n	36	36	18	17		
Mean (SD)	79.63 (27.920)	7.41 (28.852)	71.30 (26.695)	11.76 (24.127)		
Median	100.00	0.00	66.67	16.67		
Min, Max	0.0, 100.0	-66.7, 83.3	0.0, 100.0	-33.3, 50.0		

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Modified Randomized Set

Social Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	33	33	16	16	49	49
Mean (SD)	79.80 (26.925)	6.06 (29.703)	66.67 (31.623)	7.29 (33.316)	75.51 (28.888)	6.46 (30.584)
Median	100.00	0.00	75.00	16.67	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 83.3	0.0, 100.0	-100.0, 50.0	0.0, 100.0	-100.0, 83.3
Week 56						
n	23	23	15	14	38	37
Mean (SD)	84.06 (22.178)	13.04 (26.570)	75.56 (19.787)	10.71 (29.676)	80.70 (21.409)	12.16 (27.400)
Median	100.00	0.00	66.67	8.33	83.33	0.00
Min, Max	33.3, 100.0	-33.3, 83.3	33.3, 100.0	-50.0, 66.7	33.3, 100.0	-50.0, 83.3

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Social Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	14	14	9	8	23	22
Mean (SD)	88.10 (22.100)	20.24 (27.095)	85.19 (15.466)	12.50 (14.773)	86.96 (19.434)	17.42 (23.275)
Median	100.00	16.67	83.33	16.67	100.00	16.67
Min, Max	33.3, 100.0	-16.7, 83.3	66.7, 100.0	-16.7, 33.3	33.3, 100.0	-16.7, 83.3
Week 88						
n	8	8	6	6	14	14
Mean (SD)	91.67 (15.430)	20.83 (27.817)	83.33 (18.257)	25.00 (22.973)	88.10 (16.575)	22.62 (24.985)
Median	100.00	16.67	83.33	16.67	100.00	16.67
Min, Max	66.7, 100.0	0.0, 83.3	66.7, 100.0	0.0, 66.7	66.7, 100.0	0.0, 83.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Social Functioning

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	2	2	5	5
Mean (SD)	100.00 (0.000)	11.11 (19.245)	83.33 (23.570)	58.33 (11.785)	93.33 (14.907)	30.00 (29.814)
Median	100.00	0.00	83.33	58.33	100.00	33.33
Min, Max	100.0, 100.0	0.0, 33.3	66.7, 100.0	50.0, 66.7	66.7, 100.0	0.0, 66.7

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Global Health Status/QoL

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean (SD)	63.02 (14.583)		56.94 (15.290)		61.36 (14.671)	
Median	58.33		50.00		58.33	
Min, Max	41.7, 83.3		41.7, 83.3		41.7, 83.3	
Week 2						
n	16	16	5	5		
Mean (SD)	64.58 (13.088)	1.56 (16.164)	61.67 (16.245)	6.67 (21.570)		
Median	66.67	0.00	66.67	0.00		
Min, Max	33.3, 83.3	-25.0, 33.3	41.7, 83.3	-16.7, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Global Health Status/QoL

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	16	16	6	6		
Mean (SD)	69.27 (11.674)	6.25 (17.078)	56.94 (8.193)	0.00 (11.785)		
Median	66.67	0.00	54.17	0.00		
Min, Max	50.0, 91.7	-16.7, 33.3	50.0, 66.7	-16.7, 16.7		
Week 8						
n	13	13	5	5		
Mean (SD)	67.31 (20.259)	5.13 (23.457)	55.00 (23.274)	0.00 (26.352)		
Median	66.67	0.00	66.67	0.00		
Min, Max	16.7, 91.7	-33.3, 41.7	16.7, 75.0	-33.3, 33.3		

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Modified Randomized Set

Global Health Status/QoL

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	13	13	6	6		
Mean (SD)	70.51 (15.817)	8.33 (17.678)	62.50 (16.457)	5.56 (22.153)		
Median	66.67	8.33	66.67	4.17		
Min, Max	33.3, 91.7	-16.7, 41.7	33.3, 83.3	-16.7, 41.7		
Week 24						
n	13	13	6	6		
Mean (SD)	66.67 (17.678)	4.49 (20.586)	61.11 (14.593)	4.17 (17.280)		
Median	66.67	0.00	66.67	4.17		
Min, Max	16.7, 83.3	-33.3, 33.3	33.3, 75.0	-16.7, 25.0		

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Modified Randomized Set

Global Health Status/QoL

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	13	13	5	5	18	18
Mean (SD)	67.31 (15.761)	6.41 (16.013)	60.00 (13.693)	8.33 (20.412)	65.28 (15.192)	6.94 (16.728)
Median	66.67	0.00	66.67	16.67	66.67	4.17
Min, Max	33.3, 83.3	-16.7, 33.3	41.7, 75.0	-16.7, 33.3	33.3, 83.3	-16.7, 33.3
Week 56						
n	11	11	2	2	13	13
Mean (SD)	63.64 (17.588)	1.52 (16.167)	50.00 (0.000)	-8.33 (11.785)	61.54 (16.853)	0.00 (15.590)
Median	66.67	0.00	50.00	-8.33	50.00	0.00
Min, Max	33.3, 83.3	-16.7, 33.3	50.0, 50.0	-16.7, 0.0	33.3, 83.3	-16.7, 33.3

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Global Health Status/QoL

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	6	6	2	2	8	8
Mean (SD)	72.22 (8.607)	1.39 (14.353)	41.67 (11.785)	-16.67 (0.000)	64.58 (16.517)	-3.13 (14.731)
Median	66.67	0.00	41.67	-16.67	66.67	-4.17
Min, Max	66.7, 83.3	-16.7, 25.0	33.3, 50.0	-16.7, -16.7	33.3, 83.3	-16.7, 25.0
Week 88						
n	6	6	2	2	8	8
Mean (SD)	68.06 (8.193)	-2.78 (15.516)	33.33 (23.570)	-25.00 (11.785)	59.38 (19.638)	-8.33 (17.252)
Median	66.67	-4.17	33.33	-25.00	66.67	-12.50
Min, Max	58.3, 83.3	-16.7, 25.0	16.7, 50.0	-33.3, -16.7	16.7, 83.3	-33.3, 25.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Global Health Status/QoL

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	58.33 (NA)	0.00 (NA)			58.33 (NA)	0.00 (NA)
Median	58.33	0.00			58.33	0.00
Min, Max	58.3, 58.3	0.0, 0.0			58.3, 58.3	0.0, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Physical Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean (SD)	75.00 (14.907)		66.67 (15.776)		72.73 (15.245)	
Median	73.33		63.33		70.00	
Min, Max	53.3, 100.0		46.7, 93.3		46.7, 100.0	
Week 2						
n	16	16	5	5		
Mean (SD)	75.83 (16.125)	0.83 (10.853)	68.00 (16.600)	0.00 (4.714)		
Median	76.67	0.00	66.67	0.00		
Min, Max	53.3, 100.0	-20.0, 20.0	46.7, 93.3	-6.7, 6.7		

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Modified Randomized Set

Physical Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	16	16	6	6		
Mean (SD)	84.17 (12.852)	9.17 (14.376)	65.56 (18.094)	-1.11 (8.861)		
Median	86.67	3.33	66.67	0.00		
Min, Max	60.0, 100.0	-6.7, 46.7	46.7, 93.3	-13.3, 13.3		
Week 8						
n	13	13	5	5		
Mean (SD)	74.87 (17.460)	2.56 (14.021)	65.33 (28.829)	-2.67 (13.824)		
Median	80.00	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-26.7, 26.7	20.0, 100.0	-26.7, 6.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Physical Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	13	13	6	6		
Mean (SD)	81.54 (11.911)	7.18 (12.311)	61.11 (24.374)	-5.56 (12.232)		
Median	86.67	6.67	63.33	0.00		
Min, Max	60.0, 100.0	-6.7, 33.3	20.0, 93.3	-26.7, 6.7		
Week 24						
n	13	13	6	6		
Mean (SD)	80.00 (14.402)	5.64 (13.566)	66.67 (19.322)	0.00 (7.303)		
Median	86.67	0.00	56.67	0.00		
Min, Max	53.3, 100.0	-6.7, 33.3	53.3, 100.0	-6.7, 6.7		

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Modified Randomized Set

Physical Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	13	13	5	5	18	18
Mean (SD)	80.00 (15.154)	6.67 (13.333)	60.00 (32.998)	-6.67 (16.997)	74.44 (22.434)	2.96 (15.206)
Median	80.00	0.00	53.33	-6.67	80.00	0.00
Min, Max	53.3, 100.0	-13.3, 26.7	20.0, 100.0	-26.7, 13.3	20.0, 100.0	-26.7, 26.7
Week 56						
n	11	11	2	2	13	13
Mean (SD)	76.97 (15.596)	3.03 (13.780)	70.00 (32.998)	-6.67 (9.428)	75.90 (17.329)	1.54 (13.376)
Median	80.00	0.00	70.00	-6.67	80.00	0.00
Min, Max	53.3, 100.0	-20.0, 20.0	46.7, 93.3	-13.3, 0.0	46.7, 100.0	-20.0, 20.0

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Modified Randomized Set

Physical Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	6	6	2	2	8	8
Mean (SD)	78.89 (15.444)	3.33 (10.954)	40.00 (47.140)	-13.33 (37.712)	69.17 (28.494)	-0.83 (18.666)
Median	80.00	6.67	40.00	-13.33	76.67	6.67
Min, Max	53.3, 100.0	-13.3, 13.3	6.7, 73.3	-40.0, 13.3	6.7, 100.0	-40.0, 13.3
Week 88						
n	6	6	2	2	8	8
Mean (SD)	86.67 (11.926)	11.11 (13.109)	43.33 (14.142)	-10.00 (4.714)	75.83 (23.077)	5.83 (14.880)
Median	90.00	10.00	43.33	-10.00	83.33	6.67
Min, Max	66.7, 100.0	-6.7, 33.3	33.3, 53.3	-13.3, -6.7	33.3, 100.0	-13.3, 33.3

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Physical Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	73.33 (NA)	13.33 (NA)			73.33 (NA)	13.33 (NA)
Median	73.33	13.33			73.33	13.33
Min, Max	73.3, 73.3	13.3, 13.3			73.3, 73.3	13.3, 13.3

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Role Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean (SD)	75.00 (21.943)		69.44 (30.581)		73.48 (23.937)	
Median	83.33		75.00		83.33	
Min, Max	33.3, 100.0		33.3, 100.0		33.3, 100.0	
Week 2						
n	15	15	4	4		
Mean (SD)	75.56 (23.458)	0.00 (19.920)	83.33 (19.245)	-4.17 (20.972)		
Median	83.33	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-50.0, 33.3	66.7, 100.0	-33.3, 16.7		

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Modified Randomized Set

Role Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	16	16	5	5		
Mean (SD)	84.38 (18.727)	9.38 (23.546)	76.67 (19.003)	0.00 (11.785)		
Median	83.33	8.33	83.33	0.00		
Min, Max	33.3, 100.0	-50.0, 50.0	50.0, 100.0	-16.7, 16.7		
Week 8						
n	13	13	4	4		
Mean (SD)	78.21 (20.844)	3.85 (20.586)	70.83 (20.972)	-16.67 (13.608)		
Median	83.33	0.00	66.67	-16.67		
Min, Max	33.3, 100.0	-33.3, 33.3	50.0, 100.0	-33.3, 0.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Role Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	13	13	5	5		
Mean (SD)	82.05 (17.296)	6.41 (25.036)	73.33 (9.129)	-3.33 (32.059)		
Median	83.33	16.67	66.67	-16.67		
Min, Max	50.0, 100.0	-33.3, 33.3	66.7, 83.3	-33.3, 50.0		
Week 24						
n	13	13	6	6		
Mean (SD)	71.79 (31.465)	-3.85 (25.598)	75.00 (17.480)	5.56 (34.427)		
Median	83.33	0.00	75.00	8.33		
Min, Max	0.0, 100.0	-50.0, 33.3	50.0, 100.0	-33.3, 50.0		

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Role Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	13	13	5	5	18	18
Mean (SD)	75.64 (25.107)	-1.28 (23.035)	50.00 (40.825)	-13.33 (13.944)	68.52 (31.253)	-4.63 (21.241)
Median	66.67	0.00	66.67	-16.67	66.67	0.00
Min, Max	16.7, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 0.0	0.0, 100.0	-66.7, 33.3
Week 56						
n	11	11	2	2	13	13
Mean (SD)	71.21 (29.899)	-6.06 (30.067)	83.33 (23.570)	16.67 (23.570)	73.08 (28.495)	-2.56 (29.538)
Median	66.67	0.00	83.33	16.67	66.67	0.00
Min, Max	0.0, 100.0	-83.3, 16.7	66.7, 100.0	0.0, 33.3	0.0, 100.0	-83.3, 33.3

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Role Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	6	6	1	1	7	7
Mean (SD)	75.00 (20.412)	2.78 (12.546)	100.00 (NA)	66.67 (NA)	78.57 (20.893)	11.90 (26.726)
Median	66.67	0.00	100.00	66.67	66.67	0.00
Min, Max	50.0, 100.0	-16.7, 16.7	100.0, 100.0	66.7, 66.7	50.0, 100.0	-16.7, 66.7
Week 88						
n	6	6	2	2	8	8
Mean (SD)	83.33 (21.082)	11.11 (20.184)	41.67 (35.355)	8.33 (35.355)	72.92 (29.463)	10.42 (21.708)
Median	91.67	0.00	41.67	8.33	75.00	0.00
Min, Max	50.0, 100.0	0.0, 50.0	16.7, 66.7	-16.7, 33.3	16.7, 100.0	-16.7, 50.0

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Role Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	50.00 (NA)	16.67 (NA)			50.00 (NA)	16.67 (NA)
Median	50.00	16.67			50.00	16.67
Min, Max	50.0, 50.0	16.7, 16.7			50.0, 50.0	16.7, 16.7

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Emotional Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean (SD)	82.29 (12.500)		90.28 (9.742)		84.47 (12.143)	
Median	79.17		91.67		83.33	
Min, Max	66.7, 100.0		75.0, 100.0		66.7, 100.0	
Week 2						
n	15	15	5	5		
Mean (SD)	81.11 (15.258)	-1.11 (9.894)	81.67 (10.865)	-10.00 (10.865)		
Median	83.33	0.00	75.00	-8.33		
Min, Max	58.3, 100.0	-16.7, 16.7	75.0, 100.0	-25.0, 0.0		

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Emotional Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	16	16	6	6		
Mean (SD)	88.54 (12.865)	6.25 (11.180)	83.33 (14.907)	-6.94 (16.173)		
Median	91.67	0.00	83.33	-8.33		
Min, Max	66.7, 100.0	-8.3, 33.3	66.7, 100.0	-33.3, 16.7		
Week 8						
n	13	13	5	5		
Mean (SD)	83.33 (18.321)	3.21 (15.038)	81.67 (9.129)	-10.00 (3.727)		
Median	83.33	0.00	83.33	-8.33		
Min, Max	50.0, 100.0	-25.0, 33.3	66.7, 91.7	-16.7, -8.3		

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Emotional Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	13	13	6	6		
Mean (SD)	89.10 (12.903)	5.77 (12.903)	77.78 (19.484)	-12.50 (22.205)		
Median	91.67	8.33	75.00	-12.50		
Min, Max	58.3, 100.0	-16.7, 33.3	50.0, 100.0	-50.0, 16.7		
Week 24						
n	13	13	6	6		
Mean (SD)	88.46 (13.411)	5.13 (12.972)	86.11 (13.608)	-4.17 (11.487)		
Median	91.67	0.00	87.50	-8.33		
Min, Max	58.3, 100.0	-16.7, 33.3	66.7, 100.0	-16.7, 16.7		

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Table GERMANY.2.5.11.3

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Modified Randomized Set

Emotional Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	13	13	5	5	18	18
Mean (SD)	88.46 (11.044)	5.77 (14.587)	71.67 (12.638)	-18.33 (9.129)	83.80 (13.550)	-0.93 (17.123)
Median	91.67	0.00	66.67	-25.00	87.50	0.00
Min, Max	66.7, 100.0	-16.7, 33.3	58.3, 91.7	-25.0, -8.3	58.3, 100.0	-25.0, 33.3
Week 56						
n	11	11	2	2	13	13
Mean (SD)	85.61 (17.516)	4.55 (17.225)	87.50 (17.678)	8.33 (11.785)	85.90 (16.800)	5.13 (16.151)
Median	91.67	0.00	87.50	8.33	91.67	0.00
Min, Max	50.0, 100.0	-25.0, 25.0	75.0, 100.0	0.0, 16.7	50.0, 100.0	-25.0, 25.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Emotional Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	6	6	2	2	8	8
Mean (SD)	90.28 (16.173)	9.72 (17.808)	87.50 (17.678)	-4.17 (29.463)	89.58 (15.269)	6.25 (19.796)
Median	95.83	8.33	87.50	-4.17	95.83	8.33
Min, Max	58.3, 100.0	-16.7, 33.3	75.0, 100.0	-25.0, 16.7	58.3, 100.0	-25.0, 33.3
Week 88						
n	6	6	2	2	8	8
Mean (SD)	87.50 (13.693)	6.94 (11.076)	83.33 (23.570)	-8.33 (35.355)	86.46 (14.731)	3.13 (17.783)
Median	91.67	8.33	83.33	-8.33	91.67	8.33
Min, Max	66.7, 100.0	-8.3, 25.0	66.7, 100.0	-33.3, 16.7	66.7, 100.0	-33.3, 25.0

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Table GERMANY.2.5.11.3

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Emotional Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	83.33 (NA)	8.33 (NA)			83.33 (NA)	8.33 (NA)
Median	83.33	8.33			83.33	8.33
Min, Max	83.3, 83.3	8.3, 8.3			83.3, 83.3	8.3, 8.3

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Cognitive Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean (SD)	82.29 (17.710)		77.78 (25.092)		81.06 (19.447)	
Median	83.33		83.33		83.33	
Min, Max	50.0, 100.0		33.3, 100.0		33.3, 100.0	
Week 2						
n	15	15	5	5		
Mean (SD)	78.89 (18.330)	-3.33 (9.344)	63.33 (38.006)	-10.00 (22.361)		
Median	83.33	0.00	66.67	0.00		
Min, Max	50.0, 100.0	-16.7, 16.7	0.0, 100.0	-33.3, 16.7		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Cognitive Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	16	16	6	6		
Mean (SD)	84.38 (14.232)	2.08 (13.437)	63.89 (34.021)	-13.89 (22.153)		
Median	83.33	0.00	66.67	-16.67		
Min, Max	66.7, 100.0	-16.7, 33.3	0.0, 100.0	-33.3, 16.7		
Week 8						
n	13	13	5	5		
Mean (SD)	79.49 (19.429)	-1.28 (17.296)	70.00 (24.721)	-3.33 (18.257)		
Median	83.33	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 33.3	33.3, 100.0	-33.3, 16.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Cognitive Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	13	13	6	6		
Mean (SD)	80.77 (19.059)	0.00 (15.215)	69.44 (28.707)	-8.33 (20.412)		
Median	83.33	0.00	75.00	-16.67		
Min, Max	33.3, 100.0	-16.7, 33.3	16.7, 100.0	-33.3, 16.7		
Week 24						
n	13	13	6	6		
Mean (SD)	79.49 (23.721)	-1.28 (19.792)	75.00 (22.973)	-2.78 (19.484)		
Median	83.33	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-33.3, 33.3	33.3, 100.0	-33.3, 16.7		

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Modified Randomized Set

Cognitive Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	13	13	5	5	18	18
Mean (SD)	80.77 (21.350)	-1.28 (19.792)	56.67 (32.489)	-20.00 (18.257)	74.07 (26.335)	-6.48 (20.721)
Median	83.33	0.00	66.67	-33.33	83.33	0.00
Min, Max	33.3, 100.0	-50.0, 16.7	0.0, 83.3	-33.3, 0.0	0.0, 100.0	-50.0, 16.7
Week 56						
n	11	11	2	2	13	13
Mean (SD)	80.30 (20.841)	-3.03 (16.361)	83.33 (0.000)	0.00 (23.570)	80.77 (19.059)	-2.56 (16.452)
Median	83.33	0.00	83.33	0.00	83.33	0.00
Min, Max	50.0, 100.0	-33.3, 33.3	83.3, 83.3	-16.7, 16.7	50.0, 100.0	-33.3, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Cognitive Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	6	6	2	2	8	8
Mean (SD)	94.44 (8.607)	5.56 (13.608)	58.33 (58.926)	-8.33 (11.785)	85.42 (28.781)	2.08 (13.909)
Median	100.00	0.00	58.33	-8.33	100.00	0.00
Min, Max	83.3, 100.0	0.0, 33.3	16.7, 100.0	-16.7, 0.0	16.7, 100.0	-16.7, 33.3
Week 88						
n	6	6	2	2	8	8
Mean (SD)	94.44 (8.607)	5.56 (8.607)	41.67 (58.926)	-25.00 (11.785)	81.25 (33.850)	-2.08 (16.517)
Median	100.00	0.00	41.67	-25.00	91.67	0.00
Min, Max	83.3, 100.0	0.0, 16.7	0.0, 83.3	-33.3, -16.7	0.0, 100.0	-33.3, 16.7

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Cognitive Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	66.67 (NA)	0.00 (NA)			66.67 (NA)	0.00 (NA)
Median	66.67	0.00			66.67	0.00
Min, Max	66.7, 66.7	0.0, 0.0			66.7, 66.7	0.0, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Social Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		6		22	
Mean (SD)	84.38 (16.632)		97.22 (6.804)		87.88 (15.585)	
Median	83.33		100.00		100.00	
Min, Max	50.0, 100.0		83.3, 100.0		50.0, 100.0	
Week 2						
n	16	16	4	4		
Mean (SD)	87.50 (16.667)	3.13 (10.918)	91.67 (16.667)	-4.17 (8.333)		
Median	100.00	0.00	100.00	0.00		
Min, Max	50.0, 100.0	-16.7, 33.3	66.7, 100.0	-16.7, 0.0		

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Modified Randomized Set

Social Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	16	16	5	5		
Mean (SD)	90.63 (16.066)	6.25 (10.319)	93.33 (9.129)	-3.33 (13.944)		
Median	100.00	0.00	100.00	0.00		
Min, Max	50.0, 100.0	0.0, 33.3	83.3, 100.0	-16.7, 16.7		
Week 8						
n	13	13	4	4		
Mean (SD)	84.62 (19.792)	1.28 (14.372)	91.67 (9.623)	-4.17 (8.333)		
Median	83.33	0.00	91.67	0.00		
Min, Max	33.3, 100.0	-33.3, 16.7	83.3, 100.0	-16.7, 0.0		

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Modified Randomized Set

Social Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	13	13	6	6		
Mean (SD)	92.31 (11.004)	5.13 (10.507)	80.56 (34.021)	-16.67 (33.333)		
Median	100.00	0.00	100.00	0.00		
Min, Max	66.7, 100.0	0.0, 33.3	16.7, 100.0	-83.3, 0.0		
Week 24						
n	13	13	5	5		
Mean (SD)	88.46 (15.789)	1.28 (15.901)	90.00 (14.907)	-6.67 (19.003)		
Median	100.00	0.00	100.00	0.00		
Min, Max	66.7, 100.0	-33.3, 33.3	66.7, 100.0	-33.3, 16.7		

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Social Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	13	13	5	5	18	18
Mean (SD)	80.77 (19.059)	-6.41 (17.398)	43.33 (46.547)	-53.33 (50.553)	70.37 (32.618)	-19.44 (35.815)
Median	83.33	0.00	33.33	-66.67	83.33	0.00
Min, Max	50.0, 100.0	-50.0, 16.7	0.0, 100.0	-100.0, 0.0	0.0, 100.0	-100.0, 16.7
Week 56						
n	11	11	2	2	13	13
Mean (SD)	83.33 (21.082)	-3.03 (17.979)	91.67 (11.785)	-8.33 (11.785)	84.62 (19.792)	-3.85 (16.879)
Median	83.33	0.00	91.67	-8.33	83.33	0.00
Min, Max	33.3, 100.0	-50.0, 16.7	83.3, 100.0	-16.7, 0.0	33.3, 100.0	-50.0, 16.7

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Social Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	6	6	2	2	8	8
Mean (SD)	88.89 (13.608)	8.33 (20.412)	58.33 (58.926)	-41.67 (58.926)	81.25 (28.781)	-4.17 (36.460)
Median	91.67	0.00	58.33	-41.67	91.67	0.00
Min, Max	66.7, 100.0	-16.7, 33.3	16.7, 100.0	-83.3, 0.0	16.7, 100.0	-83.3, 33.3
Week 88						
n	6	6	2	2	8	8
Mean (SD)	80.56 (16.387)	0.00 (10.541)	66.67 (0.000)	-33.33 (0.000)	77.08 (15.269)	-8.33 (17.817)
Median	75.00	0.00	66.67	-33.33	66.67	0.00
Min, Max	66.7, 100.0	-16.7, 16.7	66.7, 66.7	-33.3, -33.3	66.7, 100.0	-33.3, 16.7

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Table GERMANY.2.5.11.3

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Social Functioning

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	0	0	1	1
Mean (SD)	66.67 (NA)	-33.33 (NA)			66.67 (NA)	-33.33 (NA)
Median	66.67	-33.33			66.67	-33.33
Min, Max	66.7, 66.7	-33.3, -33.3			66.7, 66.7	-33.3, -33.3

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Table GERMANY.2.5.11.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Global Health Status/QoL

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	25		12		37	
Mean (SD)	55.00 (19.543)		59.03 (12.542)		56.31 (17.503)	
Median	50.00		66.67		58.33	
Min, Max	16.7, 83.3		33.3, 75.0		16.7, 83.3	
Week 2						
n	25	25	13	12		
Mean (SD)	65.33 (18.893)	10.33 (13.878)	56.41 (12.799)	-4.86 (12.542)		
Median	66.67	16.67	58.33	0.00		
Min, Max	33.3, 100.0	-16.7, 33.3	33.3, 83.3	-25.0, 16.7		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Global Health Status/QoL

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	25	25	13	12		
Mean (SD)	65.33 (20.081)	10.33 (16.715)	55.77 (19.059)	-5.56 (14.794)		
Median	66.67	16.67	50.00	0.00		
Min, Max	25.0, 91.7	-16.7, 41.7	33.3, 83.3	-41.7, 16.7		
Week 8						
n	19	19	8	7		
Mean (SD)	68.42 (23.989)	12.28 (21.222)	57.29 (23.332)	-10.71 (19.670)		
Median	75.00	16.67	62.50	0.00		
Min, Max	16.7, 100.0	-33.3, 50.0	16.7, 83.3	-33.3, 16.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Global Health Status/QoL

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	23	23	13	12		
Mean (SD)	70.29 (15.856)	16.30 (12.175)	57.05 (17.953)	-3.47 (14.416)		
Median	66.67	16.67	50.00	-4.17		
Min, Max	41.7, 100.0	-8.3, 33.3	33.3, 83.3	-25.0, 16.7		
Week 24						
n	20	20	13	12		
Mean (SD)	68.75 (18.511)	12.50 (13.653)	67.31 (13.803)	8.33 (10.050)		
Median	66.67	16.67	66.67	8.33		
Min, Max	33.3, 100.0	-8.3, 33.3	33.3, 83.3	-8.3, 25.0		

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Modified Randomized Set

Global Health Status/QoL

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	12	12	30	30
Mean (SD)	65.74 (20.590)	10.19 (18.426)	64.58 (22.226)	5.56 (16.412)	65.28 (20.886)	8.33 (17.508)
Median	66.67	12.50	66.67	4.17	66.67	8.33
Min, Max	33.3, 100.0	-41.7, 33.3	16.7, 100.0	-16.7, 33.3	16.7, 100.0	-41.7, 33.3
Week 56						
n	12	12	11	10	23	22
Mean (SD)	63.19 (20.244)	6.25 (17.453)	62.12 (15.076)	0.83 (20.582)	62.68 (17.565)	3.79 (18.674)
Median	62.50	0.00	66.67	0.00	66.67	0.00
Min, Max	33.3, 91.7	-16.7, 33.3	33.3, 83.3	-25.0, 41.7	33.3, 91.7	-25.0, 41.7

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Table GERMANY.2.5.11.4
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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Global Health Status/QoL

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	8	7	16	15
Mean (SD)	69.79 (17.783)	5.21 (14.042)	64.58 (19.288)	0.00 (17.347)	67.19 (18.122)	2.78 (15.323)
Median	66.67	4.17	66.67	0.00	66.67	0.00
Min, Max	33.3, 91.7	-16.7, 25.0	33.3, 91.7	-16.7, 25.0	33.3, 91.7	-16.7, 25.0
Week 88						
n	6	6	5	5	11	11
Mean (SD)	75.00 (12.910)	5.56 (16.387)	61.67 (26.087)	1.67 (27.259)	68.94 (20.101)	3.79 (20.871)
Median	75.00	8.33	66.67	0.00	66.67	0.00
Min, Max	58.3, 91.7	-16.7, 25.0	16.7, 83.3	-33.3, 41.7	16.7, 91.7	-33.3, 41.7

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Table GERMANY.2.5.11.4
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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Global Health Status/QoL

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	2	2	3	3
Mean (SD)	58.33 (NA)	0.00 (NA)	79.17 (5.893)	25.00 (23.570)	72.22 (12.729)	16.67 (22.048)
Median	58.33	0.00	79.17	25.00	75.00	8.33
Min, Max	58.3, 58.3	0.0, 0.0	75.0, 83.3	8.3, 41.7	58.3, 83.3	0.0, 41.7

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Table GERMANY.2.5.11.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Physical Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	25		12		37	
Mean (SD)	71.47 (16.161)		66.67 (18.202)		69.91 (16.750)	
Median	66.67		66.67		66.67	
Min, Max	33.3, 100.0		26.7, 86.7		26.7, 100.0	
Week 2						
n	25	25	13	12		
Mean (SD)	77.07 (18.962)	5.60 (10.660)	65.64 (14.618)	-2.22 (9.570)		
Median	80.00	0.00	66.67	0.00		
Min, Max	26.7, 100.0	-13.3, 26.7	40.0, 86.7	-13.3, 20.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Physical Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	25	25	13	12		
Mean (SD)	83.47 (17.226)	12.00 (13.053)	62.56 (22.033)	-6.11 (14.343)		
Median	86.67	13.33	60.00	-3.33		
Min, Max	40.0, 100.0	-20.0, 46.7	26.7, 100.0	-26.7, 13.3		
Week 8						
n	19	19	8	7		
Mean (SD)	85.96 (17.483)	11.93 (14.501)	64.17 (22.236)	-5.71 (13.569)		
Median	86.67	13.33	70.00	0.00		
Min, Max	33.3, 100.0	-26.7, 40.0	20.0, 86.7	-26.7, 13.3		

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Table GERMANY.2.5.11.4
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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Physical Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	23	23	13	12		
Mean (SD)	82.90 (17.271)	12.17 (11.833)	61.54 (20.755)	-6.67 (17.979)		
Median	86.67	13.33	66.67	-3.33		
Min, Max	26.7, 100.0	-6.7, 33.3	20.0, 93.3	-33.3, 20.0		
Week 24						
n	20	20	13	12		
Mean (SD)	82.33 (17.475)	10.67 (16.387)	78.97 (20.700)	11.67 (11.055)		
Median	86.67	13.33	86.67	13.33		
Min, Max	40.0, 100.0	-33.3, 40.0	40.0, 100.0	-6.7, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Physical Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	12	12	30	30
Mean (SD)	79.63 (22.343)	8.89 (15.509)	72.78 (23.348)	6.11 (14.622)	76.89 (22.607)	7.78 (14.967)
Median	83.33	13.33	73.33	6.67	80.00	13.33
Min, Max	13.3, 100.0	-26.7, 26.7	20.0, 100.0	-26.7, 26.7	13.3, 100.0	-26.7, 26.7
Week 56						
n	12	12	11	10	23	22
Mean (SD)	81.11 (19.034)	9.44 (10.429)	76.36 (15.878)	6.67 (16.630)	78.84 (17.367)	8.18 (13.323)
Median	86.67	13.33	80.00	6.67	80.00	13.33
Min, Max	46.7, 100.0	-6.7, 20.0	53.3, 100.0	-20.0, 33.3	46.7, 100.0	-20.0, 33.3

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Modified Randomized Set

Physical Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	8	7	16	15
Mean (SD)	84.17 (16.306)	11.67 (9.920)	68.33 (28.002)	-0.95 (19.409)	76.25 (23.598)	5.78 (15.910)
Median	90.00	13.33	70.00	0.00	80.00	6.67
Min, Max	53.3, 100.0	-6.7, 26.7	6.7, 93.3	-40.0, 20.0	6.7, 100.0	-40.0, 26.7
Week 88						
n	6	6	5	5	11	11
Mean (SD)	91.11 (13.109)	15.56 (11.675)	68.00 (23.758)	4.00 (18.012)	80.61 (21.386)	10.30 (15.308)
Median	96.67	16.67	66.67	0.00	86.67	6.67
Min, Max	66.7, 100.0	0.0, 33.3	33.3, 93.3	-13.3, 33.3	33.3, 100.0	-13.3, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Physical Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	2	2	3	3
Mean (SD)	73.33 (NA)	13.33 (NA)	90.00 (4.714)	20.00 (18.856)	84.44 (10.184)	17.78 (13.878)
Median	73.33	13.33	90.00	20.00	86.67	13.33
Min, Max	73.3, 73.3	13.3, 13.3	86.7, 93.3	6.7, 33.3	73.3, 93.3	6.7, 33.3

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Role Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	25		12		37	
Mean (SD)	64.67 (23.234)		56.94 (27.023)		62.16 (24.422)	
Median	66.67		66.67		66.67	
Min, Max	16.7, 100.0		16.7, 100.0		16.7, 100.0	
Week 2						
n	24	24	12	11		
Mean (SD)	72.92 (29.819)	8.33 (26.006)	63.89 (26.432)	3.03 (12.513)		
Median	83.33	16.67	66.67	0.00		
Min, Max	0.0, 100.0	-50.0, 50.0	16.7, 100.0	-16.7, 33.3		

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Role Functioning

Europe

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	25	25	12	11		
Mean (SD)	74.67 (31.225)	10.00 (29.659)	65.28 (27.941)	3.03 (31.463)		
Median	83.33	16.67	66.67	0.00		
Min, Max	0.0, 100.0	-66.7, 50.0	16.7, 100.0	-33.3, 83.3		
Week 8						
n	19	19	7	6		
Mean (SD)	82.46 (23.223)	14.91 (24.149)	61.90 (23.002)	-5.56 (17.213)		
Median	100.00	16.67	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 50.0	33.3, 100.0	-33.3, 16.7		

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Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	23	23	12	11		
Mean (SD)	77.54 (24.929)	13.04 (22.449)	59.72 (32.920)	0.00 (35.746)		
Median	66.67	16.67	66.67	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 66.7		
Week 24						
n	20	20	13	12		
Mean (SD)	67.50 (37.258)	2.50 (33.014)	70.51 (34.797)	13.89 (43.712)		
Median	83.33	0.00	83.33	25.00		
Min, Max	0.0, 100.0	-66.7, 50.0	0.0, 100.0	-100.0, 66.7		

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Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	12	12	30	30
Mean (SD)	71.30 (32.743)	5.56 (30.250)	66.67 (36.237)	9.72 (32.144)	69.44 (33.643)	7.22 (30.539)
Median	66.67	0.00	75.00	16.67	66.67	8.33
Min, Max	0.0, 100.0	-66.7, 50.0	0.0, 100.0	-50.0, 50.0	0.0, 100.0	-66.7, 50.0
Week 56						
n	12	12	11	10	23	22
Mean (SD)	66.67 (35.533)	1.39 (35.146)	77.27 (13.484)	20.00 (28.109)	71.74 (27.264)	9.85 (32.797)
Median	66.67	0.00	66.67	16.67	66.67	16.67
Min, Max	0.0, 100.0	-83.3, 50.0	66.7, 100.0	-33.3, 66.7	0.0, 100.0	-83.3, 66.7

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Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	7	6	15	14
Mean (SD)	75.00 (25.198)	14.58 (24.296)	69.05 (22.420)	16.67 (21.082)	72.22 (23.288)	15.48 (22.134)
Median	75.00	16.67	66.67	25.00	66.67	16.67
Min, Max	33.3, 100.0	-33.3, 50.0	50.0, 100.0	-16.7, 33.3	33.3, 100.0	-33.3, 50.0
Week 88						
n	6	6	5	5	11	11
Mean (SD)	91.67 (20.412)	30.56 (19.484)	60.00 (27.889)	6.67 (34.561)	77.27 (28.159)	19.70 (28.692)
Median	100.00	33.33	66.67	0.00	83.33	16.67
Min, Max	50.0, 100.0	0.0, 50.0	16.7, 83.3	-16.7, 66.7	16.7, 100.0	-16.7, 66.7

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Modified Randomized Set

Role Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	2	2	3	3
Mean (SD)	50.00 (NA)	16.67 (NA)	83.33 (23.570)	33.33 (23.570)	72.22 (25.459)	27.78 (19.245)
Median	50.00	16.67	83.33	33.33	66.67	16.67
Min, Max	50.0, 50.0	16.7, 16.7	66.7, 100.0	16.7, 50.0	50.0, 100.0	16.7, 50.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Emotional Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	25		12		37	
Mean (SD)	80.67 (21.613)		72.92 (24.905)		78.15 (22.682)	
Median	83.33		83.33		83.33	
Min, Max	0.0, 100.0		8.3, 100.0		0.0, 100.0	
Week 2						
n	24	24	13	12		
Mean (SD)	85.42 (18.100)	4.86 (13.883)	74.36 (21.099)	-0.69 (16.839)		
Median	91.67	0.00	75.00	0.00		
Min, Max	33.3, 100.0	-16.7, 33.3	33.3, 100.0	-33.3, 25.0		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Emotional Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	25	25	13	12		
Mean (SD)	82.33 (26.278)	1.67 (23.323)	75.64 (22.685)	0.69 (20.554)		
Median	91.67	0.00	83.33	0.00		
Min, Max	0.0, 100.0	-66.7, 33.3	33.3, 100.0	-33.3, 33.3		
Week 8						
n	19	19	8	7		
Mean (SD)	90.35 (13.398)	7.89 (12.569)	91.67 (12.599)	9.52 (13.113)		
Median	100.00	8.33	100.00	16.67		
Min, Max	50.0, 100.0	-16.7, 33.3	66.7, 100.0	-16.7, 16.7		

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Emotional Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	23	23	13	12		
Mean (SD)	86.96 (19.107)	6.52 (10.037)	69.87 (27.118)	-3.47 (20.554)		
Median	91.67	8.33	75.00	0.00		
Min, Max	16.7, 100.0	-8.3, 25.0	0.0, 100.0	-50.0, 16.7		
Week 24						
n	20	20	13	12		
Mean (SD)	82.92 (28.418)	3.33 (20.305)	85.26 (12.339)	13.19 (15.674)		
Median	91.67	0.00	83.33	8.33		
Min, Max	0.0, 100.0	-66.7, 33.3	58.3, 100.0	-8.3, 50.0		

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Emotional Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	12	12	30	30
Mean (SD)	79.17 (28.332)	-0.46 (22.772)	81.94 (13.685)	9.03 (22.879)	80.28 (23.313)	3.33 (22.911)
Median	87.50	4.17	75.00	4.17	83.33	4.17
Min, Max	0.0, 100.0	-66.7, 25.0	66.7, 100.0	-25.0, 66.7	0.0, 100.0	-66.7, 66.7
Week 56						
n	12	12	11	10	23	22
Mean (SD)	77.08 (32.976)	1.39 (30.325)	78.03 (17.189)	7.50 (29.775)	77.54 (26.043)	4.17 (29.519)
Median	95.83	12.50	83.33	0.00	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	50.0, 100.0	-16.7, 75.0	0.0, 100.0	-66.7, 75.0

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Emotional Functioning

Europe

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	8	7	16	15
Mean (SD)	86.46 (17.216)	1.04 (18.601)	77.08 (19.796)	5.95 (17.817)	81.77 (18.564)	3.33 (17.762)
Median	95.83	4.17	75.00	8.33	83.33	8.33
Min, Max	58.3, 100.0	-33.3, 25.0	41.7, 100.0	-25.0, 33.3	41.7, 100.0	-33.3, 33.3
Week 88						
n	6	6	5	5	11	11
Mean (SD)	91.67 (12.910)	11.11 (11.386)	76.67 (18.066)	11.67 (45.491)	84.85 (16.591)	11.36 (29.878)
Median	95.83	12.50	83.33	0.00	91.67	8.33
Min, Max	66.7, 100.0	-8.3, 25.0	50.0, 91.7	-33.3, 83.3	50.0, 100.0	-33.3, 83.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Emotional Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	2	2	3	3
Mean (SD)	83.33 (NA)	8.33 (NA)	62.50 (41.248)	25.00 (82.496)	69.44 (31.549)	19.44 (59.122)
Median	83.33	8.33	62.50	25.00	83.33	8.33
Min, Max	83.3, 83.3	8.3, 8.3	33.3, 91.7	-33.3, 83.3	33.3, 91.7	-33.3, 83.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Cognitive Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	25		12		37	
Mean (SD)	80.00 (19.837)		70.83 (18.970)		77.03 (19.779)	
Median	83.33		66.67		83.33	
Min, Max	16.7, 100.0		33.3, 100.0		16.7, 100.0	
Week 2						
n	24	24	13	12		
Mean (SD)	82.64 (18.701)	2.78 (16.051)	67.95 (29.235)	-4.17 (20.257)		
Median	83.33	0.00	83.33	0.00		
Min, Max	50.0, 100.0	-16.7, 33.3	0.0, 100.0	-33.3, 33.3		

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Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	25	25	13	12		
Mean (SD)	82.00 (22.006)	2.00 (18.207)	65.38 (30.778)	-6.94 (20.669)		
Median	83.33	0.00	66.67	-16.67		
Min, Max	16.7, 100.0	-50.0, 33.3	0.0, 100.0	-33.3, 33.3		
Week 8						
n	19	19	8	7		
Mean (SD)	85.09 (18.337)	4.39 (15.558)	77.08 (23.465)	7.14 (13.113)		
Median	83.33	0.00	83.33	16.67		
Min, Max	33.3, 100.0	-33.3, 33.3	33.3, 100.0	-16.7, 16.7		

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Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	23	23	13	12		
Mean (SD)	86.23 (17.154)	6.52 (13.045)	71.79 (27.542)	0.00 (18.803)		
Median	83.33	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-16.7, 33.3	16.7, 100.0	-33.3, 33.3		
Week 24						
n	20	20	13	12		
Mean (SD)	79.17 (31.933)	0.00 (22.942)	78.21 (22.958)	6.94 (15.006)		
Median	100.00	0.00	83.33	8.33		
Min, Max	0.0, 100.0	-66.7, 33.3	33.3, 100.0	-16.7, 33.3		

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Cognitive Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	12	12	30	30
Mean (SD)	74.07 (29.826)	-0.93 (23.895)	69.44 (30.011)	-1.39 (18.060)	72.22 (29.470)	-1.11 (21.412)
Median	83.33	0.00	75.00	0.00	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 33.3
Week 56						
n	12	12	11	10	23	22
Mean (SD)	76.39 (30.533)	4.17 (25.746)	72.73 (29.129)	0.00 (23.570)	74.64 (29.245)	2.27 (24.286)
Median	91.67	0.00	83.33	0.00	83.33	0.00
Min, Max	16.7, 100.0	-50.0, 33.3	0.0, 100.0	-50.0, 33.3	0.0, 100.0	-50.0, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Cognitive Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	7	6	15	14
Mean (SD)	87.50 (14.773)	6.25 (17.678)	73.81 (28.637)	5.56 (13.608)	81.11 (22.596)	5.95 (15.480)
Median	91.67	0.00	83.33	8.33	83.33	0.00
Min, Max	66.7, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 16.7	16.7, 100.0	-16.7, 33.3
Week 88						
n	6	6	5	5	11	11
Mean (SD)	94.44 (8.607)	16.67 (14.907)	56.67 (40.139)	-6.67 (27.889)	77.27 (32.722)	6.06 (23.889)
Median	100.00	16.67	66.67	0.00	83.33	0.00
Min, Max	83.3, 100.0	0.0, 33.3	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 33.3

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Cognitive Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	2	2	3	3
Mean (SD)	66.67 (NA)	0.00 (NA)	58.33 (35.355)	-8.33 (35.355)	61.11 (25.459)	-5.56 (25.459)
Median	66.67	0.00	58.33	-8.33	66.67	0.00
Min, Max	66.7, 66.7	0.0, 0.0	33.3, 83.3	-33.3, 16.7	33.3, 83.3	-33.3, 16.7

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Social Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	25		12		37	
Mean (SD)	78.67 (21.257)		59.72 (32.920)		72.52 (26.706)	
Median	83.33		66.67		83.33	
Min, Max	33.3, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	25	25	12	11		
Mean (SD)	84.00 (20.115)	5.33 (21.365)	61.11 (28.721)	1.52 (21.672)		
Median	100.00	0.00	66.67	0.00		
Min, Max	50.0, 100.0	-50.0, 66.7	0.0, 100.0	-33.3, 50.0		

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Social Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	25	25	12	11		
Mean (SD)	82.67 (22.812)	4.00 (18.181)	58.33 (23.028)	-1.52 (25.226)		
Median	100.00	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 33.3	0.0, 100.0	-50.0, 50.0		
Week 8						
n	19	19	7	6		
Mean (SD)	86.84 (21.212)	7.89 (22.477)	80.95 (17.817)	11.11 (17.213)		
Median	100.00	16.67	66.67	16.67		
Min, Max	33.3, 100.0	-50.0, 33.3	66.7, 100.0	-16.7, 33.3		

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Social Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	23	23	13	12		
Mean (SD)	85.51 (20.904)	6.52 (18.627)	52.56 (31.802)	-11.11 (32.824)		
Median	100.00	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 33.3	0.0, 100.0	-83.3, 33.3		
Week 24						
n	20	20	12	11		
Mean (SD)	78.33 (33.377)	-1.67 (26.435)	66.67 (30.151)	7.58 (20.226)		
Median	100.00	0.00	66.67	16.67		
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-33.3, 33.3		

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Social Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	18	12	12	30	30
Mean (SD)	75.93 (29.826)	-5.56 (24.254)	55.56 (35.770)	-4.17 (48.265)	67.78 (33.314)	-5.00 (35.056)
Median	83.33	0.00	66.67	8.33	75.00	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-100.0, 50.0	0.0, 100.0	-100.0, 50.0
Week 56						
n	12	12	11	10	23	22
Mean (SD)	83.33 (25.624)	1.39 (15.006)	69.70 (19.462)	10.00 (34.427)	76.81 (23.430)	5.30 (25.400)
Median	100.00	0.00	66.67	8.33	83.33	0.00
Min, Max	33.3, 100.0	-33.3, 16.7	33.3, 100.0	-50.0, 66.7	33.3, 100.0	-50.0, 66.7

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Social Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	7	6	15	14
Mean (SD)	83.33 (23.570)	2.08 (13.909)	69.05 (26.227)	-8.33 (39.087)	76.67 (25.040)	-2.38 (26.840)
Median	91.67	0.00	66.67	8.33	83.33	0.00
Min, Max	33.3, 100.0	-16.7, 16.7	16.7, 100.0	-83.3, 16.7	16.7, 100.0	-83.3, 16.7
Week 88						
n	6	6	5	5	11	11
Mean (SD)	97.22 (6.804)	8.33 (13.944)	73.33 (14.907)	13.33 (36.132)	86.36 (16.361)	10.61 (25.025)
Median	100.00	16.67	66.67	16.67	100.00	16.67
Min, Max	83.3, 100.0	-16.7, 16.7	66.7, 100.0	-33.3, 66.7	66.7, 100.0	-33.3, 66.7

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Social Functioning

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	2	2	3	3
Mean (SD)	66.67 (NA)	-33.33 (NA)	83.33 (23.570)	58.33 (11.785)	77.78 (19.245)	27.78 (53.576)
Median	66.67	-33.33	83.33	58.33	66.67	50.00
Min, Max	66.7, 66.7	-33.3, -33.3	66.7, 100.0	50.0, 66.7	66.7, 100.0	-33.3, 66.7

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Global Health Status/QoL

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	60.94 (20.570)		51.39 (21.271)		58.33 (20.959)	
Median	66.67		50.00		58.33	
Min, Max	25.0, 100.0		16.7, 91.7		16.7, 100.0	
Week 2						
n	32	32	12	11		
Mean (SD)	62.76 (19.741)	1.82 (19.253)	60.42 (18.844)	12.12 (20.871)		
Median	66.67	0.00	66.67	16.67		
Min, Max	16.7, 100.0	-50.0, 41.7	25.0, 83.3	-16.7, 50.0		

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Table GERMANY.2.5.11.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Global Health Status/QoL

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	32	32	12	12		
Mean (SD)	67.97 (19.296)	7.03 (16.547)	56.25 (24.650)	4.86 (17.210)		
Median	66.67	0.00	54.17	4.17		
Min, Max	25.0, 100.0	-16.7, 50.0	25.0, 100.0	-16.7, 41.7		
Week 8						
n	26	26	11	11		
Mean (SD)	66.35 (20.747)	6.41 (23.606)	59.85 (23.219)	9.85 (19.656)		
Median	66.67	8.33	66.67	16.67		
Min, Max	25.0, 100.0	-58.3, 41.7	16.7, 83.3	-16.7, 41.7		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Global Health Status/QoL

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	29	29	12	12		
Mean (SD)	69.54 (21.161)	8.91 (17.242)	68.06 (14.579)	16.67 (16.667)		
Median	75.00	8.33	66.67	16.67		
Min, Max	33.3, 100.0	-16.7, 41.7	50.0, 91.7	-16.7, 41.7		
Week 24						
n	29	29	11	11		
Mean (SD)	68.97 (20.278)	8.33 (19.416)	65.15 (24.386)	12.88 (19.495)		
Median	66.67	8.33	66.67	16.67		
Min, Max	16.7, 100.0	-33.3, 41.7	16.7, 100.0	-16.7, 41.7		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Global Health Status/QoL

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	28	28	9	9	37	37
Mean (SD)	70.54 (19.041)	10.12 (14.761)	60.19 (25.610)	7.41 (21.018)	68.02 (20.927)	9.46 (16.216)
Median	66.67	8.33	66.67	16.67	66.67	8.33
Min, Max	33.3, 100.0	-16.7, 33.3	16.7, 100.0	-33.3, 33.3	16.7, 100.0	-33.3, 33.3
Week 56						
n	22	22	6	6	28	28
Mean (SD)	68.94 (17.851)	7.95 (15.952)	59.72 (30.008)	1.39 (23.224)	66.96 (20.723)	6.55 (17.474)
Median	66.67	4.17	66.67	-4.17	66.67	0.00
Min, Max	41.7, 100.0	-16.7, 41.7	8.3, 83.3	-25.0, 33.3	8.3, 100.0	-25.0, 41.7

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Global Health Status/QoL

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	4	4	16	16
Mean (SD)	69.44 (15.215)	9.72 (27.486)	58.33 (31.914)	6.25 (38.112)	66.67 (19.954)	8.85 (29.102)
Median	66.67	8.33	66.67	4.17	66.67	8.33
Min, Max	41.7, 100.0	-50.0, 41.7	16.7, 83.3	-33.3, 50.0	16.7, 100.0	-50.0, 50.0
Week 88						
n	8	8	3	3	11	11
Mean (SD)	73.96 (17.501)	10.42 (15.905)	61.11 (19.245)	8.33 (22.048)	70.45 (18.014)	9.85 (16.591)
Median	75.00	12.50	50.00	16.67	66.67	16.67
Min, Max	41.7, 100.0	-16.7, 33.3	50.0, 83.3	-16.7, 25.0	41.7, 100.0	-16.7, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Global Health Status/QoL

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	0	0	3	3
Mean (SD)	88.89 (19.245)	11.11 (19.245)			88.89 (19.245)	11.11 (19.245)
Median	100.00	0.00			100.00	0.00
Min, Max	66.7, 100.0	0.0, 33.3			66.7, 100.0	0.0, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Physical Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	74.79 (20.790)		63.89 (23.348)		71.82 (21.798)	
Median	80.00		66.67		73.33	
Min, Max	33.3, 100.0		26.7, 100.0		26.7, 100.0	
Week 2						
n	31	31	12	11		
Mean (SD)	80.43 (16.683)	6.24 (13.215)	67.78 (22.353)	4.85 (13.692)		
Median	86.67	0.00	66.67	0.00		
Min, Max	40.0, 100.0	-20.0, 46.7	26.7, 100.0	-13.3, 40.0		

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Modified Randomized Set

Physical Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	32	32	12	12		
Mean (SD)	84.79 (13.988)	10.00 (15.885)	71.67 (19.720)	7.78 (17.941)		
Median	86.67	6.67	76.67	3.33		
Min, Max	53.3, 100.0	-6.7, 60.0	33.3, 100.0	-13.3, 53.3		
Week 8						
n	26	26	11	11		
Mean (SD)	81.79 (14.181)	8.21 (15.003)	75.15 (19.571)	10.91 (16.404)		
Median	80.00	0.00	73.33	6.67		
Min, Max	53.3, 100.0	-13.3, 46.7	33.3, 100.0	-6.7, 46.7		

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Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	29	29	12	12		
Mean (SD)	86.90 (13.509)	10.34 (14.969)	71.11 (19.763)	7.22 (14.896)		
Median	86.67	6.67	73.33	3.33		
Min, Max	53.3, 100.0	-6.7, 46.7	40.0, 100.0	-13.3, 46.7		
Week 24						
n	29	29	11	11		
Mean (SD)	88.28 (12.428)	11.72 (17.335)	76.36 (24.651)	9.09 (15.570)		
Median	93.33	6.67	80.00	6.67		
Min, Max	53.3, 100.0	-6.7, 60.0	26.7, 100.0	-6.7, 40.0		

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Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	28	28	9	9	37	37
Mean (SD)	86.43 (13.393)	9.52 (15.227)	74.81 (28.043)	2.96 (14.948)	83.60 (18.298)	7.93 (15.222)
Median	86.67	6.67	86.67	0.00	86.67	6.67
Min, Max	60.0, 100.0	-13.3, 53.3	33.3, 100.0	-20.0, 26.7	33.3, 100.0	-20.0, 53.3
Week 56						
n	22	22	6	6	28	28
Mean (SD)	84.55 (14.891)	9.09 (17.033)	84.44 (20.074)	5.56 (12.232)	84.52 (15.719)	8.33 (15.986)
Median	86.67	6.67	90.00	6.67	86.67	6.67
Min, Max	53.3, 100.0	-20.0, 60.0	46.7, 100.0	-13.3, 20.0	46.7, 100.0	-20.0, 60.0

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Physical Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	4	4	17	17
Mean (SD)	84.62 (12.877)	9.23 (20.099)	88.33 (11.386)	18.33 (3.333)	85.49 (12.301)	11.37 (17.914)
Median	86.67	6.67	90.00	20.00	86.67	13.33
Min, Max	60.0, 100.0	-13.3, 60.0	73.3, 100.0	13.3, 20.0	60.0, 100.0	-13.3, 60.0
Week 88						
n	8	8	3	3	11	11
Mean (SD)	85.83 (13.063)	7.50 (9.718)	84.44 (26.943)	13.33 (17.638)	85.45 (16.280)	9.09 (11.652)
Median	86.67	6.67	100.00	20.00	86.67	6.67
Min, Max	60.0, 100.0	-6.7, 26.7	53.3, 100.0	-6.7, 26.7	53.3, 100.0	-6.7, 26.7

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Table GERMANY.2.5.11.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Physical Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	0	0	3	3
Mean (SD)	97.78 (3.849)	6.67 (6.667)			97.78 (3.849)	6.67 (6.667)
Median	100.00	6.67			100.00	6.67
Min, Max	93.3, 100.0	0.0, 13.3			93.3, 100.0	0.0, 13.3

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Table GERMANY.2.5.11.4
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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Role Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	69.27 (30.559)		69.44 (29.158)		69.32 (29.845)	
Median	66.67		66.67		66.67	
Min, Max	0.0, 100.0		16.7, 100.0		0.0, 100.0	
Week 2						
n	31	31	12	11		
Mean (SD)	76.88 (27.446)	8.60 (21.888)	72.22 (29.588)	1.52 (21.672)		
Median	83.33	0.00	75.00	0.00		
Min, Max	0.0, 100.0	-16.7, 66.7	0.0, 100.0	-33.3, 33.3		

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Modified Randomized Set

Role Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	32	32	12	12		
Mean (SD)	86.46 (14.930)	17.19 (25.570)	70.83 (25.746)	1.39 (20.669)		
Median	83.33	0.00	83.33	0.00		
Min, Max	50.0, 100.0	-16.7, 83.3	16.7, 100.0	-33.3, 50.0		
Week 8						
n	26	26	11	11		
Mean (SD)	80.77 (19.260)	14.74 (23.724)	71.21 (28.955)	-1.52 (34.524)		
Median	83.33	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-16.7, 83.3	16.7, 100.0	-66.7, 66.7		

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Role Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	29	29	12	12		
Mean (SD)	85.63 (19.783)	15.52 (25.947)	77.78 (21.711)	8.33 (33.710)		
Median	100.00	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-33.3, 66.7	33.3, 100.0	-33.3, 66.7		
Week 24						
n	29	29	11	11		
Mean (SD)	84.48 (18.328)	14.37 (26.624)	78.79 (23.677)	6.06 (30.067)		
Median	83.33	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-33.3, 83.3	33.3, 100.0	-33.3, 50.0		

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Role Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	28	28	9	9	37	37
Mean (SD)	85.71 (16.174)	13.10 (23.288)	70.37 (34.134)	0.00 (14.434)	81.98 (22.353)	9.91 (22.034)
Median	100.00	0.00	66.67	0.00	100.00	0.00
Min, Max	66.7, 100.0	-16.7, 66.7	16.7, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 66.7
Week 56						
n	22	22	6	6	28	28
Mean (SD)	78.79 (18.674)	5.30 (23.223)	88.89 (17.213)	11.11 (27.217)	80.95 (18.545)	6.55 (23.718)
Median	66.67	0.00	100.00	16.67	75.00	0.00
Min, Max	33.3, 100.0	-33.3, 66.7	66.7, 100.0	-33.3, 33.3	33.3, 100.0	-33.3, 66.7

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Role Functioning

Rest of World

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	4	4	17	17
Mean (SD)	84.62 (29.235)	15.38 (27.606)	91.67 (9.623)	25.00 (34.694)	86.27 (25.844)	17.65 (28.547)
Median	100.00	0.00	91.67	25.00	100.00	0.00
Min, Max	0.0, 100.0	-16.7, 66.7	83.3, 100.0	-16.7, 66.7	0.0, 100.0	-16.7, 66.7
Week 88						
n	8	8	3	3	11	11
Mean (SD)	81.25 (24.296)	0.00 (8.909)	88.89 (19.245)	22.22 (19.245)	83.33 (22.361)	6.06 (15.407)
Median	91.67	0.00	100.00	33.33	100.00	0.00
Min, Max	33.3, 100.0	-16.7, 16.7	66.7, 100.0	0.0, 33.3	33.3, 100.0	-16.7, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Role Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	0	0	3	3
Mean (SD)	100.00 (0.000)	0.00 (0.000)			100.00 (0.000)	0.00 (0.000)
Median	100.00	0.00			100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0			100.0, 100.0	0.0, 0.0

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Modified Randomized Set

Emotional Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	78.91 (23.282)		75.00 (24.873)		77.84 (23.498)	
Median	83.33		79.17		83.33	
Min, Max	0.0, 100.0		8.3, 100.0		0.0, 100.0	
Week 2						
n	32	32	12	11		
Mean (SD)	83.59 (18.511)	4.69 (16.925)	73.61 (22.143)	3.03 (13.577)		
Median	91.67	0.00	79.17	0.00		
Min, Max	41.7, 100.0	-25.0, 58.3	33.3, 100.0	-16.7, 25.0		

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Emotional Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	32	32	12	12		
Mean (SD)	85.42 (19.167)	6.51 (14.624)	79.17 (22.332)	4.17 (12.563)		
Median	91.67	0.00	83.33	0.00		
Min, Max	8.3, 100.0	-8.3, 58.3	25.0, 100.0	-8.3, 33.3		
Week 8						
n	26	26	11	11		
Mean (SD)	81.41 (18.455)	5.77 (20.517)	77.27 (23.597)	3.03 (13.056)		
Median	83.33	0.00	83.33	0.00		
Min, Max	41.7, 100.0	-25.0, 58.3	16.7, 100.0	-8.3, 33.3		

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Emotional Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	29	29	12	12		
Mean (SD)	87.64 (18.449)	8.33 (16.815)	79.17 (19.624)	4.17 (15.284)		
Median	100.00	0.00	75.00	0.00		
Min, Max	41.7, 100.0	-16.7, 66.7	33.3, 100.0	-16.7, 33.3		
Week 24						
n	29	29	11	11		
Mean (SD)	86.21 (17.434)	6.90 (17.687)	75.76 (22.501)	0.00 (19.720)		
Median	100.00	0.00	83.33	0.00		
Min, Max	41.7, 100.0	-25.0, 66.7	33.3, 100.0	-33.3, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Emotional Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	28	28	9	9	37	37
Mean (SD)	89.29 (15.525)	8.04 (15.628)	72.22 (23.570)	-1.85 (18.993)	85.14 (18.956)	5.63 (16.788)
Median	100.00	4.17	66.67	-8.33	91.67	0.00
Min, Max	41.7, 100.0	-25.0, 58.3	25.0, 100.0	-25.0, 33.3	25.0, 100.0	-25.0, 58.3
Week 56						
n	22	22	6	6	28	28
Mean (SD)	85.23 (17.429)	4.92 (16.398)	80.56 (21.517)	11.11 (20.184)	84.23 (18.050)	6.25 (17.067)
Median	91.67	0.00	83.33	8.33	91.67	0.00
Min, Max	50.0, 100.0	-25.0, 50.0	41.7, 100.0	-16.7, 33.3	41.7, 100.0	-25.0, 50.0

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Emotional Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	4	4	16	16
Mean (SD)	93.75 (9.485)	8.33 (14.213)	75.00 (35.355)	14.58 (17.180)	89.06 (19.654)	9.90 (14.662)
Median	100.00	0.00	87.50	16.67	100.00	4.17
Min, Max	75.0, 100.0	-8.3, 33.3	25.0, 100.0	-8.3, 33.3	25.0, 100.0	-8.3, 33.3
Week 88						
n	8	8	3	3	11	11
Mean (SD)	90.63 (12.939)	3.13 (9.898)	77.78 (38.490)	25.00 (8.333)	87.12 (21.201)	9.09 (13.670)
Median	95.83	0.00	100.00	25.00	100.00	0.00
Min, Max	66.7, 100.0	-8.3, 25.0	33.3, 100.0	16.7, 33.3	33.3, 100.0	-8.3, 33.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Emotional Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	0	0	3	3
Mean (SD)	100.00 (0.000)	0.00 (0.000)			100.00 (0.000)	0.00 (0.000)
Median	100.00	0.00			100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0			100.0, 100.0	0.0, 0.0

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Cognitive Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	77.60 (24.905)		69.44 (33.207)		75.38 (27.253)	
Median	83.33		75.00		83.33	
Min, Max	16.7, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	32	32	11	11		
Mean (SD)	83.33 (17.961)	5.73 (17.765)	80.30 (25.624)	13.64 (25.624)		
Median	83.33	0.00	83.33	16.67		
Min, Max	33.3, 100.0	-16.7, 66.7	16.7, 100.0	-33.3, 66.7		

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Cognitive Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	32	32	12	12		
Mean (SD)	86.46 (13.677)	8.85 (18.445)	77.78 (28.721)	8.33 (27.061)		
Median	83.33	0.00	83.33	0.00		
Min, Max	66.7, 100.0	-16.7, 50.0	0.0, 100.0	-33.3, 50.0		
Week 8						
n	26	26	11	11		
Mean (SD)	83.33 (14.907)	10.26 (19.480)	77.27 (30.067)	10.61 (25.025)		
Median	83.33	0.00	83.33	0.00		
Min, Max	50.0, 100.0	-16.7, 66.7	0.0, 100.0	-33.3, 50.0		

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Cognitive Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	29	29	12	12		
Mean (SD)	83.91 (18.084)	7.47 (17.012)	79.17 (24.746)	9.72 (26.071)		
Median	83.33	0.00	83.33	8.33		
Min, Max	33.3, 100.0	-16.7, 50.0	16.7, 100.0	-33.3, 50.0		
Week 24						
n	29	29	11	11		
Mean (SD)	84.48 (17.212)	8.05 (18.698)	78.79 (22.473)	7.58 (21.556)		
Median	83.33	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-16.7, 50.0	33.3, 100.0	-33.3, 33.3		

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Cognitive Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	28	28	9	9	37	37
Mean (SD)	87.50 (14.073)	8.93 (17.262)	72.22 (25.000)	-3.70 (21.695)	83.78 (18.209)	5.86 (18.928)
Median	91.67	0.00	66.67	0.00	83.33	0.00
Min, Max	66.7, 100.0	-33.3, 50.0	16.7, 100.0	-33.3, 33.3	16.7, 100.0	-33.3, 50.0
Week 56						
n	22	22	6	6	28	28
Mean (SD)	88.64 (14.895)	4.55 (15.585)	75.00 (37.639)	2.78 (19.484)	85.71 (21.619)	4.17 (16.117)
Median	100.00	0.00	83.33	0.00	91.67	0.00
Min, Max	50.0, 100.0	-16.7, 50.0	0.0, 100.0	-16.7, 33.3	0.0, 100.0	-16.7, 50.0

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Cognitive Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	4	4	16	16
Mean (SD)	94.44 (10.856)	6.94 (15.006)	75.00 (39.675)	8.33 (21.517)	89.58 (21.837)	7.29 (16.066)
Median	100.00	0.00	91.67	8.33	100.00	0.00
Min, Max	66.7, 100.0	0.0, 50.0	16.7, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 50.0
Week 88						
n	8	8	3	3	11	11
Mean (SD)	93.75 (12.400)	0.00 (0.000)	66.67 (44.096)	11.11 (25.459)	86.36 (25.624)	3.03 (12.513)
Median	100.00	0.00	83.33	16.67	100.00	0.00
Min, Max	66.7, 100.0	0.0, 0.0	16.7, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 33.3

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Cognitive Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	0	0	3	3
Mean (SD)	100.00 (0.000)	0.00 (0.000)			100.00 (0.000)	0.00 (0.000)
Median	100.00	0.00			100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0			100.0, 100.0	0.0, 0.0

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Social Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	32		12		44	
Mean (SD)	72.92 (28.945)		79.17 (27.639)		74.62 (28.414)	
Median	66.67		83.33		83.33	
Min, Max	0.0, 100.0		16.7, 100.0		0.0, 100.0	
Week 2						
n	32	32	12	11		
Mean (SD)	81.25 (21.895)	8.33 (23.187)	81.94 (25.084)	3.03 (17.979)		
Median	83.33	0.00	91.67	0.00		
Min, Max	33.3, 100.0	-33.3, 83.3	16.7, 100.0	-16.7, 33.3		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

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Table GERMANY.2.5.11.4
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Social Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	32	32	12	12		
Mean (SD)	83.85 (19.622)	10.94 (23.035)	86.11 (24.447)	6.94 (20.669)		
Median	91.67	0.00	100.00	0.00		
Min, Max	33.3, 100.0	-16.7, 83.3	16.7, 100.0	-16.7, 50.0		
Week 8						
n	26	26	11	11		
Mean (SD)	85.90 (18.069)	11.54 (22.983)	80.30 (25.624)	3.03 (17.979)		
Median	91.67	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-16.7, 83.3	16.7, 100.0	-16.7, 33.3		

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Social Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	29	29	12	12		
Mean (SD)	87.36 (17.624)	13.79 (21.853)	84.72 (22.983)	5.56 (14.794)		
Median	100.00	0.00	100.00	0.00		
Min, Max	33.3, 100.0	0.0, 83.3	33.3, 100.0	-16.7, 33.3		
Week 24						
n	29	29	11	11		
Mean (SD)	84.48 (18.328)	10.92 (24.911)	84.85 (15.731)	7.58 (28.249)		
Median	100.00	0.00	83.33	0.00		
Min, Max	50.0, 100.0	-33.3, 83.3	66.7, 100.0	-33.3, 50.0		

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Social Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	28	28	9	9	37	37
Mean (SD)	82.74 (21.024)	7.74 (28.139)	68.52 (36.747)	-11.11 (43.301)	79.28 (25.881)	3.15 (32.829)
Median	100.00	0.00	83.33	0.00	83.33	0.00
Min, Max	33.3, 100.0	-33.3, 83.3	0.0, 100.0	-100.0, 33.3	0.0, 100.0	-100.0, 83.3
Week 56						
n	22	22	6	6	28	28
Mean (SD)	84.09 (19.570)	11.36 (28.815)	91.67 (9.129)	5.56 (17.213)	85.71 (17.982)	10.12 (26.581)
Median	100.00	0.00	91.67	0.00	100.00	0.00
Min, Max	33.3, 100.0	-50.0, 83.3	83.3, 100.0	-16.7, 33.3	33.3, 100.0	-50.0, 83.3

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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Social Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	4	4	16	16
Mean (SD)	91.67 (16.667)	26.39 (27.023)	100.00 (0.000)	16.67 (13.608)	93.75 (14.751)	23.96 (24.319)
Median	100.00	33.33	100.00	16.67	100.00	25.00
Min, Max	50.0, 100.0	0.0, 83.3	100.0, 100.0	0.0, 33.3	50.0, 100.0	0.0, 83.3
Week 88						
n	8	8	3	3	11	11
Mean (SD)	79.17 (17.252)	14.58 (30.129)	88.89 (19.245)	5.56 (34.694)	81.82 (17.408)	12.12 (29.899)
Median	66.67	0.00	100.00	16.67	66.67	0.00
Min, Max	66.7, 100.0	0.0, 83.3	66.7, 100.0	-33.3, 33.3	66.7, 100.0	-33.3, 83.3

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Table GERMANY.2.5.11.4
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During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Social Functioning

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	0	0	3	3
Mean (SD)	100.00 (0.000)	11.11 (19.245)			100.00 (0.000)	11.11 (19.245)
Median	100.00	0.00			100.00	0.00
Min, Max	100.0, 100.0	0.0, 33.3			100.0, 100.0	0.0, 33.3

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Table GERMANY.2.5.11.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Global Health Status/QoL

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		9		33	
Mean (SD)	60.07 (24.324)		51.85 (18.056)		57.83 (22.816)	
Median	62.50		58.33		58.33	
Min, Max	16.7, 100.0		16.7, 66.7		16.7, 100.0	
Week 2						
n	24	24	9	8		
Mean (SD)	65.28 (21.656)	5.21 (19.005)	62.04 (11.867)	9.38 (22.903)		
Median	66.67	4.17	66.67	8.33		
Min, Max	33.3, 100.0	-41.7, 33.3	41.7, 83.3	-25.0, 50.0		

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Modified Randomized Set

Global Health Status/QoL

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	24	24	10	9		
Mean (SD)	70.49 (20.703)	10.42 (14.170)	52.50 (18.860)	-2.78 (10.206)		
Median	70.83	8.33	50.00	0.00		
Min, Max	33.3, 100.0	-16.7, 33.3	25.0, 83.3	-16.7, 8.3		
Week 8						
n	19	19	7	6		
Mean (SD)	62.72 (26.984)	2.63 (24.541)	61.90 (21.440)	8.33 (22.973)		
Median	58.33	8.33	66.67	16.67		
Min, Max	16.7, 100.0	-58.3, 33.3	33.3, 83.3	-33.3, 33.3		

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Global Health Status/QoL

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Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	10	9		
Mean (SD)	70.08 (21.921)	10.98 (16.140)	65.83 (14.933)	12.96 (20.460)		
Median	66.67	8.33	66.67	16.67		
Min, Max	33.3, 100.0	-16.7, 41.7	50.0, 83.3	-16.7, 41.7		
Week 24						
n	21	21	10	9		
Mean (SD)	70.24 (23.210)	9.13 (19.348)	55.00 (21.228)	1.85 (10.015)		
Median	75.00	8.33	62.50	0.00		
Min, Max	16.7, 100.0	-33.3, 33.3	16.7, 83.3	-8.3, 16.7		

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Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	8	8	27	27
Mean (SD)	68.86 (26.182)	7.89 (18.525)	51.04 (24.575)	-5.21 (21.333)	63.58 (26.569)	4.01 (19.932)
Median	83.33	16.67	50.00	-12.50	66.67	0.00
Min, Max	33.3, 100.0	-41.7, 33.3	16.7, 83.3	-33.3, 33.3	16.7, 100.0	-41.7, 33.3
Week 56						
n	13	13	7	6	20	19
Mean (SD)	70.51 (22.209)	7.69 (14.618)	61.90 (18.545)	1.39 (21.355)	67.50 (20.925)	5.70 (16.679)
Median	75.00	0.00	66.67	0.00	66.67	0.00
Min, Max	33.3, 100.0	-16.7, 33.3	33.3, 83.3	-25.0, 33.3	33.3, 100.0	-25.0, 33.3

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Table GERMANY.2.5.11.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Global Health Status/QoL

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	6	5	14	13
Mean (SD)	66.67 (16.060)	-1.04 (23.754)	59.72 (27.089)	-8.33 (20.412)	63.69 (20.829)	-3.85 (21.947)
Median	66.67	0.00	58.33	-16.67	66.67	0.00
Min, Max	41.7, 91.7	-50.0, 25.0	16.7, 91.7	-33.3, 16.7	16.7, 91.7	-50.0, 25.0
Week 88						
n	7	7	3	3	10	10
Mean (SD)	80.95 (14.203)	10.71 (13.363)	63.89 (12.729)	-2.78 (12.729)	75.83 (15.441)	6.67 (14.055)
Median	83.33	16.67	66.67	0.00	79.17	4.17
Min, Max	58.3, 100.0	-8.3, 25.0	50.0, 75.0	-16.7, 8.3	50.0, 100.0	-16.7, 25.0

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning. Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.11.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Global Health Status/QoL

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	75.00 (22.048)	0.00 (0.000)	75.00 (NA)	8.33 (NA)	75.00 (18.002)	2.08 (4.167)
Median	66.67	0.00	75.00	8.33	70.83	0.00
Min, Max	58.3, 100.0	0.0, 0.0	75.0, 75.0	8.3, 8.3	58.3, 100.0	0.0, 8.3

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Modified Randomized Set

Physical Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		9		33	
Mean (SD)	75.83 (17.890)		58.52 (19.938)		71.11 (19.767)	
Median	80.00		60.00		66.67	
Min, Max	33.3, 100.0		26.7, 86.7		26.7, 100.0	
Week 2						
n	24	24	9	8		
Mean (SD)	78.61 (19.260)	2.78 (10.571)	68.15 (12.373)	8.33 (15.836)		
Median	80.00	0.00	66.67	6.67		
Min, Max	26.7, 100.0	-20.0, 20.0	46.7, 86.7	-13.3, 40.0		

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Modified Randomized Set

Physical Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	24	24	10	9		
Mean (SD)	83.89 (16.201)	8.06 (10.583)	66.67 (18.053)	5.93 (11.759)		
Median	86.67	6.67	70.00	6.67		
Min, Max	53.3, 100.0	-6.7, 33.3	33.3, 86.7	-13.3, 26.7		
Week 8						
n	19	19	7	6		
Mean (SD)	78.95 (19.021)	3.51 (12.643)	73.33 (10.184)	12.22 (12.939)		
Median	80.00	0.00	73.33	10.00		
Min, Max	33.3, 100.0	-26.7, 20.0	60.0, 86.7	0.0, 33.3		

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Physical Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	10	9		
Mean (SD)	84.55 (18.099)	6.67 (10.690)	66.67 (15.072)	6.67 (10.541)		
Median	86.67	6.67	70.00	6.67		
Min, Max	26.7, 100.0	-6.7, 26.7	40.0, 86.7	-6.7, 20.0		
Week 24						
n	21	21	10	9		
Mean (SD)	85.40 (18.333)	6.98 (11.051)	69.33 (24.984)	8.89 (12.910)		
Median	93.33	6.67	76.67	13.33		
Min, Max	40.0, 100.0	-6.7, 26.7	26.7, 100.0	-6.7, 26.7		

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Physical Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	8	8	27	27
Mean (SD)	81.40 (23.683)	2.81 (13.392)	62.50 (14.666)	0.00 (14.254)	75.80 (22.882)	1.98 (13.437)
Median	93.33	0.00	60.00	-3.33	80.00	0.00
Min, Max	13.3, 100.0	-26.7, 20.0	40.0, 86.7	-20.0, 26.7	13.3, 100.0	-26.7, 26.7
Week 56						
n	13	13	7	6	20	19
Mean (SD)	78.46 (21.969)	2.56 (13.482)	69.52 (19.190)	4.44 (16.147)	75.33 (20.982)	3.16 (13.943)
Median	80.00	0.00	73.33	6.67	80.00	0.00
Min, Max	46.7, 100.0	-20.0, 20.0	46.7, 100.0	-13.3, 26.7	46.7, 100.0	-20.0, 26.7

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Modified Randomized Set

Physical Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	6	5	15	14
Mean (SD)	80.74 (16.814)	0.00 (13.333)	80.00 (13.333)	6.67 (10.541)	80.44 (15.006)	2.38 (12.433)
Median	86.67	-6.67	80.00	6.67	86.67	3.33
Min, Max	53.3, 100.0	-13.3, 20.0	60.0, 93.3	-6.7, 20.0	53.3, 100.0	-13.3, 20.0
Week 88						
n	7	7	3	3	10	10
Mean (SD)	93.33 (12.172)	11.43 (13.724)	71.11 (20.367)	0.00 (6.667)	86.67 (17.498)	8.00 (12.881)
Median	100.00	6.67	66.67	0.00	93.33	6.67
Min, Max	66.7, 100.0	-6.7, 33.3	53.3, 93.3	-6.7, 6.7	53.3, 100.0	-6.7, 33.3

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Modified Randomized Set

Physical Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	91.11 (15.396)	6.67 (6.667)	93.33 (NA)	6.67 (NA)	91.67 (12.620)	6.67 (5.443)
Median	100.00	6.67	93.33	6.67	96.67	6.67
Min, Max	73.3, 100.0	0.0, 13.3	93.3, 93.3	6.7, 6.7	73.3, 100.0	0.0, 13.3

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Modified Randomized Set

Role Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		9		33	
Mean (SD)	65.28 (25.968)		57.41 (23.733)		63.13 (25.262)	
Median	66.67		66.67		66.67	
Min, Max	16.7, 100.0		16.7, 83.3		16.7, 100.0	
Week 2						
n	24	24	9	8		
Mean (SD)	69.44 (29.760)	4.17 (19.812)	75.93 (26.498)	14.58 (13.909)		
Median	75.00	0.00	83.33	16.67		
Min, Max	0.0, 100.0	-50.0, 33.3	16.7, 100.0	0.0, 33.3		

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Role Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	24	24	10	9		
Mean (SD)	78.47 (24.808)	13.19 (23.560)	66.67 (27.217)	5.56 (32.275)		
Median	83.33	16.67	66.67	0.00		
Min, Max	0.0, 100.0	-50.0, 50.0	16.7, 100.0	-33.3, 83.3		
Week 8						
n	19	19	7	6		
Mean (SD)	75.44 (23.810)	11.40 (21.554)	71.43 (23.002)	5.56 (17.213)		
Median	83.33	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 50.0	33.3, 100.0	-16.7, 33.3		

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Modified Randomized Set

Role Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	10	9		
Mean (SD)	78.79 (23.672)	11.36 (22.647)	71.67 (23.636)	14.81 (32.750)		
Median	83.33	16.67	75.00	0.00		
Min, Max	0.0, 100.0	-33.3, 50.0	33.3, 100.0	-33.3, 66.7		
Week 24						
n	21	21	10	9		
Mean (SD)	74.60 (34.407)	7.14 (30.538)	68.33 (32.820)	11.11 (36.324)		
Median	100.00	16.67	75.00	16.67		
Min, Max	0.0, 100.0	-50.0, 50.0	0.0, 100.0	-33.3, 66.7		

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Modified Randomized Set

Role Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	8	8	27	27
Mean (SD)	74.56 (33.040)	5.26 (29.425)	58.33 (33.333)	2.08 (25.877)	69.75 (33.345)	4.32 (27.962)
Median	100.00	0.00	66.67	0.00	66.67	0.00
Min, Max	0.0, 100.0	-66.7, 50.0	0.0, 100.0	-33.3, 50.0	0.0, 100.0	-66.7, 50.0
Week 56						
n	13	13	7	6	20	19
Mean (SD)	71.79 (33.599)	2.56 (33.226)	78.57 (15.853)	30.56 (22.153)	74.17 (28.344)	11.40 (32.419)
Median	66.67	0.00	66.67	33.33	66.67	16.67
Min, Max	0.0, 100.0	-83.3, 50.0	66.7, 100.0	0.0, 66.7	0.0, 100.0	-83.3, 66.7

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Role Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	6	5	15	14
Mean (SD)	79.63 (35.136)	14.81 (21.155)	77.78 (25.092)	26.67 (30.277)	78.89 (30.516)	19.05 (24.335)
Median	100.00	16.67	83.33	33.33	100.00	16.67
Min, Max	0.0, 100.0	-16.7, 50.0	50.0, 100.0	-16.7, 66.7	0.0, 100.0	-16.7, 66.7
Week 88						
n	7	7	3	3	10	10
Mean (SD)	92.86 (18.898)	21.43 (23.002)	72.22 (9.623)	11.11 (19.245)	86.67 (18.922)	18.33 (21.445)
Median	100.00	16.67	66.67	0.00	100.00	8.33
Min, Max	50.0, 100.0	0.0, 50.0	66.7, 83.3	0.0, 33.3	50.0, 100.0	0.0, 50.0

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Role Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	83.33 (28.868)	5.56 (9.623)	100.00 (NA)	16.67 (NA)	87.50 (25.000)	8.33 (9.623)
Median	100.00	0.00	100.00	16.67	100.00	8.33
Min, Max	50.0, 100.0	0.0, 16.7	100.0, 100.0	16.7, 16.7	50.0, 100.0	0.0, 16.7

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Modified Randomized Set

Emotional Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		9		33	
Mean (SD)	80.21 (21.816)		74.07 (12.805)		78.54 (19.768)	
Median	83.33		75.00		83.33	
Min, Max	0.0, 100.0		50.0, 91.7		0.0, 100.0	
Week 2						
n	24	24	9	8		
Mean (SD)	82.99 (20.630)	2.78 (15.671)	75.93 (19.295)	0.00 (17.817)		
Median	91.67	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-25.0, 33.3	33.3, 100.0	-33.3, 16.7		

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Emotional Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	24	24	10	9		
Mean (SD)	87.50 (16.667)	7.29 (11.075)	76.67 (21.445)	0.00 (17.678)		
Median	95.83	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-8.3, 33.3	33.3, 100.0	-33.3, 25.0		
Week 8						
n	19	19	7	6		
Mean (SD)	83.33 (19.043)	1.75 (14.049)	86.90 (11.644)	6.94 (11.076)		
Median	83.33	0.00	83.33	8.33		
Min, Max	41.7, 100.0	-25.0, 25.0	66.7, 100.0	-8.3, 16.7		

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Emotional Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	10	9		
Mean (SD)	85.98 (21.111)	5.68 (9.754)	78.33 (13.721)	4.63 (11.111)		
Median	100.00	4.17	75.00	0.00		
Min, Max	16.7, 100.0	-16.7, 25.0	58.3, 100.0	-16.7, 16.7		
Week 24						
n	21	21	10	9		
Mean (SD)	85.32 (23.849)	3.97 (13.338)	79.17 (16.316)	5.56 (19.983)		
Median	91.67	0.00	79.17	8.33		
Min, Max	8.3, 100.0	-25.0, 33.3	41.7, 100.0	-33.3, 33.3		

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	8	8	27	27
Mean (SD)	85.53 (22.880)	3.95 (12.225)	71.88 (12.550)	-2.08 (17.678)	81.48 (21.099)	2.16 (13.981)
Median	100.00	0.00	66.67	0.00	83.33	0.00
Min, Max	16.7, 100.0	-25.0, 25.0	58.3, 100.0	-25.0, 25.0	16.7, 100.0	-25.0, 25.0
Week 56						
n	13	13	7	6	20	19
Mean (SD)	85.26 (22.861)	4.49 (16.879)	77.38 (19.072)	5.56 (20.861)	82.50 (21.443)	4.82 (17.637)
Median	100.00	0.00	83.33	0.00	91.67	0.00
Min, Max	33.3, 100.0	-25.0, 33.3	50.0, 100.0	-16.7, 41.7	33.3, 100.0	-25.0, 41.7

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Emotional Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	6	5	14	13
Mean (SD)	89.58 (15.269)	3.13 (11.732)	80.56 (15.516)	3.33 (9.501)	85.71 (15.480)	3.21 (10.507)
Median	95.83	0.00	75.00	0.00	91.67	0.00
Min, Max	58.3, 100.0	-16.7, 25.0	66.7, 100.0	-8.3, 16.7	58.3, 100.0	-16.7, 25.0
Week 88						
n	7	7	3	3	10	10
Mean (SD)	92.86 (12.199)	5.95 (11.501)	80.56 (26.788)	8.33 (22.048)	89.17 (17.146)	6.67 (14.055)
Median	100.00	0.00	91.67	16.67	95.83	4.17
Min, Max	66.7, 100.0	-8.3, 25.0	50.0, 100.0	-16.7, 25.0	50.0, 100.0	-16.7, 25.0

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:48:35

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Table GERMANY.2.5.11.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Emotional Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	94.44 (9.623)	2.78 (4.811)	33.33 (NA)	-33.33 (NA)	79.17 (31.549)	-6.25 (18.478)
Median	100.00	0.00	33.33	-33.33	91.67	0.00
Min, Max	83.3, 100.0	0.0, 8.3	33.3, 33.3	-33.3, -33.3	33.3, 100.0	-33.3, 8.3

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning. Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.11.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Cognitive Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		9		33	
Mean (SD)	76.39 (21.934)		74.07 (29.001)		75.76 (23.604)	
Median	83.33		66.67		83.33	
Min, Max	16.7, 100.0		16.7, 100.0		16.7, 100.0	
Week 2						
n	24	24	9	8		
Mean (SD)	77.08 (20.152)	0.69 (14.311)	68.52 (22.737)	-4.17 (31.810)		
Median	83.33	0.00	66.67	-8.33		
Min, Max	33.3, 100.0	-16.7, 33.3	33.3, 100.0	-33.3, 66.7		

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Modified Randomized Set

Cognitive Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	24	24	10	9		
Mean (SD)	82.64 (18.044)	6.25 (13.737)	65.00 (21.445)	-11.11 (26.352)		
Median	83.33	0.00	66.67	-16.67		
Min, Max	33.3, 100.0	-16.7, 33.3	33.3, 100.0	-33.3, 50.0		
Week 8						
n	19	19	7	6		
Mean (SD)	80.70 (17.798)	4.39 (18.293)	78.57 (18.545)	2.78 (28.707)		
Median	83.33	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-33.3, 33.3	50.0, 100.0	-33.3, 50.0		

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Modified Randomized Set

Cognitive Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	10	9		
Mean (SD)	81.82 (20.515)	6.06 (15.891)	76.67 (21.082)	1.85 (25.610)		
Median	83.33	0.00	75.00	0.00		
Min, Max	33.3, 100.0	-16.7, 33.3	33.3, 100.0	-33.3, 50.0		
Week 24						
n	21	21	10	9		
Mean (SD)	80.95 (27.531)	4.76 (19.821)	75.00 (21.155)	0.00 (20.412)		
Median	100.00	0.00	83.33	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	33.3, 100.0	-33.3, 33.3		

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Cognitive Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	8	8	27	27
Mean (SD)	78.95 (22.800)	5.26 (18.470)	66.67 (19.920)	-14.58 (13.909)	75.31 (22.347)	-0.62 (19.327)
Median	83.33	0.00	66.67	-16.67	66.67	0.00
Min, Max	33.3, 100.0	-50.0, 33.3	33.3, 100.0	-33.3, 0.0	33.3, 100.0	-50.0, 33.3
Week 56						
n	13	13	7	6	20	19
Mean (SD)	84.62 (24.019)	6.41 (19.882)	66.67 (33.333)	-11.11 (22.771)	78.33 (28.150)	0.88 (21.854)
Median	100.00	0.00	83.33	-8.33	83.33	0.00
Min, Max	33.3, 100.0	-33.3, 33.3	0.0, 100.0	-50.0, 16.7	0.0, 100.0	-50.0, 33.3

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Table GERMANY.2.5.11.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Cognitive Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	5	4	13	12
Mean (SD)	93.75 (12.400)	8.33 (15.430)	86.67 (13.944)	0.00 (13.608)	91.03 (12.938)	5.56 (14.794)
Median	100.00	0.00	83.33	0.00	100.00	0.00
Min, Max	66.7, 100.0	0.0, 33.3	66.7, 100.0	-16.7, 16.7	66.7, 100.0	-16.7, 33.3
Week 88						
n	7	7	3	3	10	10
Mean (SD)	97.62 (6.299)	14.29 (14.996)	61.11 (25.459)	-16.67 (16.667)	86.67 (21.943)	5.00 (20.861)
Median	100.00	16.67	66.67	-16.67	100.00	0.00
Min, Max	83.3, 100.0	0.0, 33.3	33.3, 83.3	-33.3, 0.0	33.3, 100.0	-33.3, 33.3

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Cognitive Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	88.89 (19.245)	0.00 (0.000)	33.33 (NA)	-33.33 (NA)	75.00 (31.914)	-8.33 (16.667)
Median	100.00	0.00	33.33	-33.33	83.33	0.00
Min, Max	66.7, 100.0	0.0, 0.0	33.3, 33.3	-33.3, -33.3	33.3, 100.0	-33.3, 0.0

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Modified Randomized Set

Social Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	24		9		33	
Mean (SD)	79.17 (22.116)		61.11 (33.333)		74.24 (26.382)	
Median	83.33		50.00		83.33	
Min, Max	33.3, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	24	24	9	8		
Mean (SD)	84.72 (20.214)	5.56 (23.399)	74.07 (29.001)	14.58 (20.774)		
Median	100.00	0.00	66.67	16.67		
Min, Max	50.0, 100.0	-50.0, 66.7	16.7, 100.0	-16.7, 50.0		

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Modified Randomized Set

Social Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	24	24	10	9		
Mean (SD)	86.81 (17.706)	7.64 (15.526)	71.67 (30.480)	7.41 (20.601)		
Median	100.00	0.00	75.00	0.00		
Min, Max	50.0, 100.0	-16.7, 50.0	0.0, 100.0	-16.7, 50.0		
Week 8						
n	19	19	7	6		
Mean (SD)	82.46 (20.393)	3.51 (23.293)	78.57 (15.853)	8.33 (20.412)		
Median	83.33	0.00	66.67	16.67		
Min, Max	33.3, 100.0	-50.0, 50.0	66.7, 100.0	-16.7, 33.3		

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Social Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	22	22	10	9		
Mean (SD)	84.85 (21.767)	5.30 (19.509)	70.00 (34.066)	5.56 (14.434)		
Median	100.00	0.00	75.00	0.00		
Min, Max	33.3, 100.0	-33.3, 50.0	0.0, 100.0	-16.7, 33.3		
Week 24						
n	21	21	10	9		
Mean (SD)	82.54 (27.119)	3.97 (22.301)	73.33 (28.545)	9.26 (25.154)		
Median	100.00	0.00	83.33	16.67		
Min, Max	0.0, 100.0	-33.3, 50.0	0.0, 100.0	-33.3, 50.0		

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Modified Randomized Set

Social Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	8	8	27	27
Mean (SD)	78.95 (29.836)	-5.26 (25.491)	52.08 (37.201)	-12.50 (46.930)	70.99 (33.840)	-7.41 (32.467)
Median	100.00	0.00	66.67	8.33	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-100.0, 33.3	0.0, 100.0	-100.0, 33.3
Week 56						
n	13	13	7	6	20	19
Mean (SD)	84.62 (24.964)	0.00 (19.245)	78.57 (24.934)	13.89 (26.701)	82.50 (24.468)	4.39 (22.112)
Median	100.00	0.00	83.33	16.67	100.00	0.00
Min, Max	33.3, 100.0	-50.0, 33.3	33.3, 100.0	-16.7, 50.0	33.3, 100.0	-50.0, 50.0

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Social Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	6	5	14	13
Mean (SD)	95.83 (7.715)	6.25 (15.269)	86.11 (16.387)	10.00 (9.129)	91.67 (12.659)	7.69 (12.938)
Median	100.00	0.00	91.67	16.67	100.00	0.00
Min, Max	83.3, 100.0	-16.7, 33.3	66.7, 100.0	0.0, 16.7	66.7, 100.0	-16.7, 33.3
Week 88						
n	7	7	3	3	10	10
Mean (SD)	92.86 (13.113)	4.76 (12.599)	77.78 (19.245)	0.00 (28.868)	88.33 (15.811)	3.33 (17.213)
Median	100.00	0.00	66.67	16.67	100.00	8.33
Min, Max	66.7, 100.0	-16.7, 16.7	66.7, 100.0	-33.3, 16.7	66.7, 100.0	-33.3, 16.7

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Social Functioning

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	88.89 (19.245)	-11.11 (19.245)	100.00 (NA)	50.00 (NA)	91.67 (16.667)	4.17 (34.359)
Median	100.00	0.00	100.00	50.00	100.00	0.00
Min, Max	66.7, 100.0	-33.3, 0.0	100.0, 100.0	50.0, 50.0	66.7, 100.0	-33.3, 50.0

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Global Health Status/QoL

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		15		48	
Mean (SD)	57.07 (16.812)		57.22 (17.499)		57.12 (16.842)	
Median	58.33		50.00		54.17	
Min, Max	25.0, 83.3		33.3, 91.7		25.0, 91.7	
Week 2						
n	33	33	16	15		
Mean (SD)	62.88 (17.566)	5.81 (16.599)	56.25 (17.612)	0.00 (16.060)		
Median	66.67	8.33	50.00	0.00		
Min, Max	16.7, 83.3	-50.0, 41.7	25.0, 83.3	-25.0, 33.3		

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Global Health Status/QoL

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	33	33	15	15		
Mean (SD)	64.14 (18.457)	7.07 (18.177)	58.33 (23.359)	1.11 (19.636)		
Median	66.67	0.00	66.67	0.00		
Min, Max	25.0, 91.7	-16.7, 50.0	25.0, 100.0	-41.7, 41.7		
Week 8						
n	26	26	12	12		
Mean (SD)	70.51 (17.197)	13.46 (20.286)	56.94 (24.056)	-1.39 (21.271)		
Median	70.83	16.67	66.67	0.00		
Min, Max	25.0, 91.7	-41.7, 50.0	16.7, 83.3	-33.3, 41.7		

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Global Health Status/QoL

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	15	15		
Mean (SD)	69.72 (16.592)	13.06 (15.270)	60.00 (18.420)	2.78 (16.567)		
Median	66.67	16.67	66.67	0.00		
Min, Max	33.3, 100.0	-16.7, 41.7	33.3, 91.7	-25.0, 25.0		
Week 24						
n	28	28	14	14		
Mean (SD)	67.86 (16.310)	10.71 (15.853)	74.40 (12.431)	16.07 (15.492)		
Median	66.67	16.67	66.67	16.67		
Min, Max	33.3, 100.0	-33.3, 41.7	66.7, 100.0	-16.7, 41.7		

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Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	13	13	40	40
Mean (SD)	68.52 (13.739)	11.73 (14.296)	69.87 (19.994)	13.46 (11.556)	68.96 (15.787)	12.29 (13.342)
Median	66.67	8.33	66.67	16.67	66.67	12.50
Min, Max	33.3, 100.0	-16.7, 33.3	33.3, 100.0	-8.3, 33.3	33.3, 100.0	-16.7, 33.3
Week 56						
n	21	21	10	10	31	31
Mean (SD)	64.68 (16.224)	7.14 (17.537)	60.83 (22.923)	0.83 (21.677)	63.44 (18.343)	5.11 (18.841)
Median	66.67	0.00	66.67	0.00	66.67	0.00
Min, Max	33.3, 91.7	-16.7, 41.7	8.3, 83.3	-25.0, 41.7	8.3, 91.7	-25.0, 41.7

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Global Health Status/QoL

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	6	6	18	18
Mean (SD)	71.53 (16.072)	13.89 (20.821)	65.28 (20.012)	11.11 (26.701)	69.44 (17.150)	12.96 (22.181)
Median	66.67	16.67	70.83	12.50	66.67	16.67
Min, Max	33.3, 100.0	-16.7, 41.7	33.3, 83.3	-16.7, 50.0	33.3, 100.0	-16.7, 50.0
Week 88						
n	7	7	5	5	12	12
Mean (SD)	67.86 (13.968)	5.95 (18.456)	60.00 (27.889)	8.33 (29.463)	64.58 (20.140)	6.94 (22.426)
Median	66.67	8.33	66.67	16.67	66.67	12.50
Min, Max	41.7, 83.3	-16.7, 33.3	16.7, 83.3	-33.3, 41.7	16.7, 83.3	-33.3, 41.7

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Global Health Status/QoL

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	100.00 (NA)	33.33 (NA)	83.33 (NA)	41.67 (NA)	91.67 (11.785)	37.50 (5.893)
Median	100.00	33.33	83.33	41.67	91.67	37.50
Min, Max	100.0, 100.0	33.3, 33.3	83.3, 83.3	41.7, 41.7	83.3, 100.0	33.3, 41.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Physical Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		15		48	
Mean (SD)	71.52 (19.527)		69.33 (20.440)		70.83 (19.624)	
Median	73.33		73.33		73.33	
Min, Max	33.3, 100.0		26.7, 100.0		26.7, 100.0	
Week 2						
n	32	32	16	15		
Mean (SD)	79.17 (16.656)	8.33 (12.672)	65.83 (21.344)	-2.67 (7.475)		
Median	86.67	0.00	66.67	0.00		
Min, Max	40.0, 100.0	-6.7, 46.7	26.7, 100.0	-13.3, 13.3		

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Physical Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	33	33	15	15		
Mean (SD)	84.44 (14.969)	12.93 (16.828)	67.11 (23.431)	-2.22 (19.787)		
Median	86.67	13.33	66.67	0.00		
Min, Max	40.0, 100.0	-20.0, 60.0	26.7, 100.0	-26.7, 53.3		
Week 8						
n	26	26	12	12		
Mean (SD)	86.92 (11.848)	14.36 (14.690)	68.89 (25.479)	0.56 (18.081)		
Median	86.67	13.33	73.33	0.00		
Min, Max	60.0, 100.0	-13.3, 46.7	20.0, 100.0	-26.7, 46.7		

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Physical Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	15	15		
Mean (SD)	85.56 (13.111)	14.44 (14.657)	65.78 (23.887)	-3.56 (20.137)		
Median	86.67	13.33	73.33	0.00		
Min, Max	53.3, 100.0	0.0, 46.7	20.0, 100.0	-33.3, 46.7		
Week 24						
n	28	28	14	14		
Mean (SD)	86.19 (11.888)	14.52 (19.631)	83.81 (18.437)	11.43 (13.695)		
Median	86.67	13.33	90.00	10.00		
Min, Max	53.3, 100.0	-33.3, 60.0	46.7, 100.0	-6.7, 40.0		

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Physical Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	13	13	40	40
Mean (SD)	85.43 (11.700)	13.83 (14.899)	80.51 (27.684)	7.69 (14.362)	83.83 (18.235)	11.83 (14.830)
Median	86.67	13.33	93.33	6.67	86.67	13.33
Min, Max	60.0, 100.0	-13.3, 53.3	20.0, 100.0	-26.7, 26.7	20.0, 100.0	-26.7, 53.3
Week 56						
n	21	21	10	10	31	31
Mean (SD)	86.35 (11.051)	13.33 (14.453)	86.00 (12.746)	7.33 (14.555)	86.24 (11.410)	11.40 (14.524)
Median	86.67	13.33	86.67	6.67	86.67	13.33
Min, Max	66.7, 100.0	-6.7, 60.0	66.7, 100.0	-20.0, 33.3	66.7, 100.0	-20.0, 60.0

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Modified Randomized Set

Physical Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	6	6	18	18
Mean (SD)	87.22 (11.177)	17.78 (15.131)	70.00 (33.928)	5.56 (23.633)	81.48 (22.117)	13.70 (18.643)
Median	90.00	13.33	76.67	16.67	86.67	13.33
Min, Max	66.7, 100.0	6.7, 60.0	6.7, 100.0	-40.0, 20.0	6.7, 100.0	-40.0, 60.0
Week 88						
n	7	7	5	5	12	12
Mean (SD)	82.86 (12.084)	10.48 (8.483)	76.00 (28.906)	12.00 (20.763)	80.00 (19.899)	11.11 (14.023)
Median	86.67	6.67	86.67	20.00	86.67	10.00
Min, Max	60.0, 100.0	0.0, 26.7	33.3, 100.0	-13.3, 33.3	33.3, 100.0	-13.3, 33.3

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Physical Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	93.33 (NA)	13.33 (NA)	86.67 (NA)	33.33 (NA)	90.00 (4.714)	23.33 (14.142)
Median	93.33	13.33	86.67	33.33	90.00	23.33
Min, Max	93.3, 93.3	13.3, 13.3	86.7, 86.7	33.3, 33.3	86.7, 93.3	13.3, 33.3

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Modified Randomized Set

Role Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		15		48	
Mean (SD)	68.69 (28.795)		66.67 (30.861)		68.06 (29.139)	
Median	66.67		66.67		66.67	
Min, Max	0.0, 100.0		16.7, 100.0		0.0, 100.0	
Week 2						
n	31	31	15	14		
Mean (SD)	79.57 (26.774)	11.83 (25.889)	63.33 (28.312)	-4.76 (15.231)		
Median	100.00	0.00	66.67	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 16.7		

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Role Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	33	33	14	14		
Mean (SD)	83.33 (23.570)	14.65 (30.264)	69.05 (26.840)	0.00 (21.681)		
Median	100.00	0.00	75.00	0.00		
Min, Max	0.0, 100.0	-66.7, 83.3	16.7, 100.0	-33.3, 50.0		
Week 8						
n	26	26	11	11		
Mean (SD)	85.90 (17.443)	17.31 (25.158)	65.15 (29.302)	-7.58 (33.635)		
Median	91.67	16.67	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 83.3	16.7, 100.0	-66.7, 66.7		

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Role Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	14	14		
Mean (SD)	84.44 (21.412)	16.67 (25.521)	66.67 (32.686)	-2.38 (34.503)		
Median	100.00	8.33	75.00	-8.33		
Min, Max	33.3, 100.0	-33.3, 66.7	0.0, 100.0	-66.7, 66.7		
Week 24						
n	28	28	14	14		
Mean (SD)	79.76 (23.726)	11.31 (29.416)	78.57 (28.063)	9.52 (39.067)		
Median	83.33	0.00	83.33	16.67		
Min, Max	0.0, 100.0	-66.7, 83.3	0.0, 100.0	-100.0, 50.0		

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≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	13	13	40	40
Mean (SD)	83.95 (16.331)	13.58 (23.587)	74.36 (35.103)	7.69 (26.887)	80.83 (24.034)	11.67 (24.518)
Median	83.33	0.00	100.00	0.00	91.67	0.00
Min, Max	66.7, 100.0	-16.7, 66.7	0.0, 100.0	-50.0, 33.3	0.0, 100.0	-50.0, 66.7
Week 56						
n	21	21	10	10	31	31
Mean (SD)	76.19 (20.798)	4.76 (24.234)	83.33 (15.713)	8.33 (27.499)	78.49 (19.338)	5.91 (24.925)
Median	66.67	0.00	83.33	8.33	66.67	0.00
Min, Max	33.3, 100.0	-33.3, 66.7	66.7, 100.0	-33.3, 50.0	33.3, 100.0	-33.3, 66.7

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Role Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	5	5	17	17
Mean (SD)	81.94 (21.856)	15.28 (29.694)	76.67 (19.003)	13.33 (21.731)	80.39 (20.612)	14.71 (26.927)
Median	91.67	8.33	83.33	16.67	83.33	16.67
Min, Max	33.3, 100.0	-33.3, 66.7	50.0, 100.0	-16.7, 33.3	33.3, 100.0	-33.3, 66.7
Week 88						
n	7	7	5	5	12	12
Mean (SD)	78.57 (24.934)	4.76 (15.853)	70.00 (36.132)	13.33 (36.132)	75.00 (28.868)	8.33 (25.126)
Median	83.33	0.00	83.33	0.00	83.33	0.00
Min, Max	33.3, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 66.7	16.7, 100.0	-16.7, 66.7

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Role Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	100.00 (NA)	0.00 (NA)	66.67 (NA)	50.00 (NA)	83.33 (23.570)	25.00 (35.355)
Median	100.00	0.00	66.67	50.00	83.33	25.00
Min, Max	100.0, 100.0	0.0, 0.0	66.7, 66.7	50.0, 50.0	66.7, 100.0	0.0, 50.0

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Emotional Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		15		48	
Mean (SD)	79.29 (23.117)		73.89 (29.692)		77.60 (25.156)	
Median	83.33		83.33		83.33	
Min, Max	0.0, 100.0		8.3, 100.0		0.0, 100.0	
Week 2						
n	32	32	16	15		
Mean (SD)	85.42 (16.396)	6.25 (15.554)	72.92 (22.669)	1.67 (14.156)		
Median	91.67	0.00	75.00	0.00		
Min, Max	50.0, 100.0	-8.3, 58.3	33.3, 100.0	-25.0, 25.0		

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Emotional Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	33	33	15	15		
Mean (SD)	81.57 (25.749)	2.27 (22.942)	77.78 (23.288)	3.89 (16.627)		
Median	91.67	0.00	83.33	0.00		
Min, Max	0.0, 100.0	-66.7, 58.3	25.0, 100.0	-33.3, 33.3		
Week 8						
n	26	26	12	12		
Mean (SD)	86.54 (15.470)	10.26 (19.048)	81.25 (24.650)	4.86 (14.416)		
Median	91.67	8.33	87.50	4.17		
Min, Max	58.3, 100.0	-25.0, 58.3	16.7, 100.0	-16.7, 33.3		

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Modified Randomized Set

Emotional Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	15	15		
Mean (SD)	88.33 (16.753)	8.89 (16.657)	71.67 (28.833)	-2.22 (21.238)		
Median	100.00	4.17	75.00	0.00		
Min, Max	41.7, 100.0	-8.3, 66.7	0.0, 100.0	-50.0, 33.3		
Week 24						
n	28	28	14	14		
Mean (SD)	84.52 (21.602)	6.55 (22.031)	82.14 (19.573)	7.74 (18.335)		
Median	91.67	0.00	87.50	4.17		
Min, Max	0.0, 100.0	-66.7, 66.7	33.3, 100.0	-16.7, 50.0		

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Emotional Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	13	13	40	40
Mean (SD)	85.19 (21.350)	5.25 (22.781)	81.41 (21.288)	8.33 (23.323)	83.96 (21.131)	6.25 (22.704)
Median	91.67	8.33	91.67	8.33	91.67	8.33
Min, Max	0.0, 100.0	-66.7, 58.3	25.0, 100.0	-25.0, 66.7	0.0, 100.0	-66.7, 66.7
Week 56						
n	21	21	10	10	31	31
Mean (SD)	80.56 (24.907)	3.17 (24.927)	80.00 (18.509)	10.83 (29.410)	80.38 (22.725)	5.65 (26.210)
Median	91.67	0.00	83.33	0.00	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 50.0	41.7, 100.0	-16.7, 75.0	0.0, 100.0	-66.7, 75.0

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Emotional Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	6	6	18	18
Mean (SD)	91.67 (12.309)	6.94 (18.746)	72.22 (31.914)	13.89 (21.517)	85.19 (22.058)	9.26 (19.363)
Median	100.00	4.17	83.33	16.67	95.83	8.33
Min, Max	66.7, 100.0	-33.3, 33.3	25.0, 100.0	-25.0, 33.3	25.0, 100.0	-33.3, 33.3
Week 88						
n	7	7	5	5	12	12
Mean (SD)	89.29 (13.363)	7.14 (11.210)	75.00 (26.352)	21.67 (43.141)	83.33 (20.101)	13.19 (28.306)
Median	91.67	8.33	83.33	25.00	91.67	8.33
Min, Max	66.7, 100.0	-8.3, 25.0	33.3, 100.0	-33.3, 83.3	33.3, 100.0	-33.3, 83.3

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Emotional Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	100.00 (NA)	0.00 (NA)	91.67 (NA)	83.33 (NA)	95.83 (5.893)	41.67 (58.926)
Median	100.00	0.00	91.67	83.33	95.83	41.67
Min, Max	100.0, 100.0	0.0, 0.0	91.7, 91.7	83.3, 83.3	91.7, 100.0	0.0, 83.3

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Cognitive Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		15		48	
Mean (SD)	80.30 (23.368)		67.78 (25.562)		76.39 (24.513)	
Median	83.33		66.67		83.33	
Min, Max	16.7, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	32	32	15	15		
Mean (SD)	87.50 (15.264)	7.29 (18.422)	76.67 (30.732)	8.89 (18.758)		
Median	91.67	0.00	83.33	16.67		
Min, Max	50.0, 100.0	-16.7, 66.7	0.0, 100.0	-33.3, 33.3		

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Cognitive Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	33	33	15	15		
Mean (SD)	85.86 (17.737)	5.56 (21.517)	75.56 (34.427)	7.78 (21.700)		
Median	83.33	0.00	83.33	0.00		
Min, Max	16.7, 100.0	-50.0, 50.0	0.0, 100.0	-33.3, 50.0		
Week 8						
n	26	26	12	12		
Mean (SD)	86.54 (14.921)	10.26 (17.687)	76.39 (31.349)	12.50 (16.088)		
Median	83.33	0.00	83.33	8.33		
Min, Max	50.0, 100.0	-16.7, 66.7	0.0, 100.0	0.0, 50.0		

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Cognitive Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	15	15		
Mean (SD)	87.22 (14.961)	7.78 (14.993)	74.44 (29.457)	6.67 (21.639)		
Median	91.67	0.00	83.33	16.67		
Min, Max	50.0, 100.0	-16.7, 50.0	16.7, 100.0	-33.3, 50.0		
Week 24						
n	28	28	14	14		
Mean (SD)	83.33 (21.754)	4.76 (21.687)	80.95 (23.440)	11.90 (15.231)		
Median	83.33	0.00	83.33	16.67		
Min, Max	0.0, 100.0	-66.7, 50.0	33.3, 100.0	-16.7, 33.3		

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Cognitive Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	13	13	40	40
Mean (SD)	84.57 (22.133)	4.94 (22.079)	73.08 (31.578)	5.13 (18.490)	80.83 (25.751)	5.00 (20.741)
Median	83.33	0.00	83.33	0.00	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 50.0	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 50.0
Week 56						
n	21	21	10	10	31	31
Mean (SD)	84.13 (21.393)	3.17 (19.450)	78.33 (30.480)	8.33 (18.002)	82.26 (24.319)	4.84 (18.853)
Median	83.33	0.00	83.33	8.33	83.33	0.00
Min, Max	16.7, 100.0	-50.0, 50.0	0.0, 100.0	-16.7, 33.3	0.0, 100.0	-50.0, 50.0

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Cognitive Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	6	6	18	18
Mean (SD)	90.28 (13.216)	5.56 (16.412)	63.89 (38.610)	11.11 (17.213)	81.48 (26.746)	7.41 (16.392)
Median	100.00	0.00	75.00	16.67	91.67	0.00
Min, Max	66.7, 100.0	-16.7, 50.0	16.7, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 50.0
Week 88						
n	7	7	5	5	12	12
Mean (SD)	90.48 (13.113)	0.00 (0.000)	60.00 (48.016)	10.00 (27.889)	77.78 (34.329)	4.17 (17.588)
Median	100.00	0.00	83.33	16.67	91.67	0.00
Min, Max	66.7, 100.0	0.0, 0.0	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 33.3

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Cognitive Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	100.00 (NA)	0.00 (NA)	83.33 (NA)	16.67 (NA)	91.67 (11.785)	8.33 (11.785)
Median	100.00	0.00	83.33	16.67	91.67	8.33
Min, Max	100.0, 100.0	0.0, 0.0	83.3, 83.3	16.7, 16.7	83.3, 100.0	0.0, 16.7

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Modified Randomized Set

Social Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	33		15		48	
Mean (SD)	72.73 (28.204)		74.44 (30.123)		73.26 (28.505)	
Median	66.67		83.33		83.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	33	33	15	14		
Mean (SD)	80.81 (21.699)	8.08 (21.699)	70.00 (29.005)	-4.76 (15.231)		
Median	83.33	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 83.3	0.0, 100.0	-33.3, 33.3		

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Social Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	33	33	14	14		
Mean (SD)	80.81 (22.868)	8.08 (24.693)	72.62 (25.826)	0.00 (24.460)		
Median	83.33	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 83.3	16.7, 100.0	-50.0, 50.0		
Week 8						
n	26	26	11	11		
Mean (SD)	89.10 (18.222)	14.74 (21.253)	81.82 (26.304)	4.55 (16.817)		
Median	100.00	8.33	100.00	0.00		
Min, Max	33.3, 100.0	-16.7, 83.3	16.7, 100.0	-16.7, 33.3		

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Social Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	30	30	15	15		
Mean (SD)	87.78 (16.914)	14.44 (20.869)	66.67 (31.497)	-7.78 (30.775)		
Median	100.00	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-16.7, 83.3	0.0, 100.0	-83.3, 33.3		
Week 24						
n	28	28	13	13		
Mean (SD)	81.55 (24.570)	7.14 (28.842)	76.92 (24.093)	6.41 (24.093)		
Median	100.00	0.00	66.67	0.00		
Min, Max	0.0, 100.0	-66.7, 83.3	16.7, 100.0	-33.3, 50.0		

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Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	27	13	13	40	40
Mean (SD)	80.86 (21.033)	8.02 (27.491)	66.67 (35.355)	-3.85 (45.722)	76.25 (26.924)	4.17 (34.333)
Median	83.33	0.00	83.33	0.00	83.33	0.00
Min, Max	33.3, 100.0	-33.3, 83.3	0.0, 100.0	-100.0, 50.0	0.0, 100.0	-100.0, 83.3
Week 56						
n	21	21	10	10	31	31
Mean (SD)	83.33 (19.720)	12.70 (27.338)	76.67 (16.102)	5.00 (30.480)	81.18 (18.630)	10.22 (28.113)
Median	100.00	0.00	75.00	0.00	83.33	0.00
Min, Max	33.3, 100.0	-33.3, 83.3	50.0, 100.0	-50.0, 66.7	33.3, 100.0	-50.0, 83.3

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Table GERMANY.2.5.11.5

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Modified Randomized Set

Social Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	5	5	17	17
Mean (SD)	83.33 (23.570)	23.61 (28.831)	73.33 (34.561)	-6.67 (46.547)	80.39 (26.507)	14.71 (36.268)
Median	100.00	25.00	83.33	16.67	100.00	16.67
Min, Max	33.3, 100.0	-16.7, 83.3	16.7, 100.0	-83.3, 33.3	16.7, 100.0	-83.3, 83.3
Week 88						
n	7	7	5	5	12	12
Mean (SD)	80.95 (17.817)	19.05 (31.074)	80.00 (18.257)	16.67 (37.268)	80.56 (17.164)	18.06 (32.144)
Median	66.67	0.00	66.67	16.67	66.67	8.33
Min, Max	66.7, 100.0	0.0, 83.3	66.7, 100.0	-33.3, 66.7	66.7, 100.0	-33.3, 83.3

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.11.5

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Social Functioning

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	100.00 (NA)	33.33 (NA)	66.67 (NA)	66.67 (NA)	83.33 (23.570)	50.00 (23.570)
Median	100.00	33.33	66.67	66.67	83.33	50.00
Min, Max	100.0, 100.0	33.3, 33.3	66.7, 66.7	66.7, 66.7	66.7, 100.0	33.3, 66.7

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Table GERMANY.2.5.11.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Global Health Status/QoL

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		15		50	
Mean (SD)	56.43 (20.220)		57.22 (19.124)		56.67 (19.706)	
Median	58.33		58.33		58.33	
Min, Max	16.7, 91.7		16.7, 91.7		16.7, 91.7	
Week 2						
n	35	35	15	15		
Mean (SD)	63.57 (17.160)	7.14 (17.403)	59.44 (12.936)	2.22 (19.787)		
Median	66.67	8.33	58.33	0.00		
Min, Max	33.3, 100.0	-41.7, 41.7	41.7, 83.3	-25.0, 50.0		

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Modified Randomized Set

Global Health Status/QoL

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	35	35	15	15		
Mean (SD)	66.43 (17.443)	10.00 (17.245)	55.56 (22.640)	-1.67 (19.720)		
Median	66.67	8.33	50.00	0.00		
Min, Max	25.0, 100.0	-16.7, 50.0	25.0, 100.0	-41.7, 41.7		
Week 8						
n	29	29	12	12		
Mean (SD)	61.49 (22.975)	3.45 (23.729)	59.03 (22.879)	0.00 (24.100)		
Median	66.67	8.33	62.50	0.00		
Min, Max	16.7, 100.0	-58.3, 41.7	16.7, 83.3	-33.3, 41.7		

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Modified Randomized Set

Global Health Status/QoL

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	32	32	15	15		
Mean (SD)	66.67 (17.454)	11.46 (16.361)	61.67 (18.312)	4.44 (20.380)		
Median	66.67	8.33	66.67	0.00		
Min, Max	33.3, 100.0	-16.7, 41.7	33.3, 91.7	-25.0, 33.3		
Week 24						
n	29	29	14	14		
Mean (SD)	67.53 (20.575)	10.63 (19.783)	67.86 (22.847)	9.52 (14.565)		
Median	66.67	16.67	66.67	8.33		
Min, Max	16.7, 100.0	-33.3, 41.7	16.7, 100.0	-16.7, 41.7		

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Modified Randomized Set

Global Health Status/QoL

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	12	12	41	41
Mean (SD)	66.09 (20.282)	9.48 (17.212)	61.81 (28.082)	2.08 (18.844)	64.84 (22.555)	7.32 (17.795)
Median	66.67	8.33	66.67	4.17	66.67	8.33
Min, Max	33.3, 100.0	-41.7, 33.3	16.7, 100.0	-33.3, 25.0	16.7, 100.0	-41.7, 33.3
Week 56						
n	21	21	10	10	31	31
Mean (SD)	67.86 (17.338)	9.52 (16.306)	63.33 (17.213)	3.33 (19.720)	66.40 (17.144)	7.53 (17.395)
Median	66.67	8.33	66.67	0.00	66.67	8.33
Min, Max	41.7, 100.0	-16.7, 41.7	33.3, 83.3	-25.0, 33.3	33.3, 100.0	-25.0, 41.7

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Table GERMANY.2.5.11.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Global Health Status/QoL

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	7	7	19	19
Mean (SD)	66.67 (16.283)	9.03 (19.930)	58.33 (25.909)	-2.38 (20.813)	63.60 (20.071)	4.82 (20.472)
Median	66.67	8.33	66.67	0.00	66.67	0.00
Min, Max	33.3, 91.7	-16.7, 41.7	16.7, 83.3	-33.3, 25.0	16.7, 91.7	-33.3, 41.7
Week 88						
n	8	8	5	5	13	13
Mean (SD)	72.92 (15.905)	8.33 (15.430)	61.67 (26.087)	-1.67 (21.570)	68.59 (20.171)	4.49 (17.878)
Median	75.00	12.50	66.67	0.00	66.67	8.33
Min, Max	41.7, 91.7	-16.7, 25.0	16.7, 83.3	-33.3, 25.0	16.7, 91.7	-33.3, 25.0

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Modified Randomized Set

Global Health Status/QoL

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	83.33 (23.570)	16.67 (23.570)	75.00 (NA)	8.33 (NA)	80.56 (17.347)	13.89 (17.347)
Median	83.33	16.67	75.00	8.33	75.00	8.33
Min, Max	66.7, 100.0	0.0, 33.3	75.0, 75.0	8.3, 8.3	66.7, 100.0	0.0, 33.3

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Modified Randomized Set

Physical Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		15		50	
Mean (SD)	70.10 (19.020)		64.44 (23.593)		68.40 (20.418)	
Median	66.67		66.67		66.67	
Min, Max	33.3, 100.0		26.7, 100.0		26.7, 100.0	
Week 2						
n	34	34	15	15		
Mean (SD)	76.67 (15.933)	7.25 (13.370)	69.33 (18.990)	4.89 (12.464)		
Median	80.00	6.67	66.67	0.00		
Min, Max	26.7, 100.0	-20.0, 46.7	33.3, 100.0	-13.3, 40.0		

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Modified Randomized Set

Physical Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	35	35	15	15		
Mean (SD)	82.67 (13.928)	12.57 (16.151)	68.89 (19.947)	4.44 (18.112)		
Median	86.67	6.67	66.67	0.00		
Min, Max	53.3, 100.0	-6.7, 60.0	33.3, 100.0	-26.7, 53.3		
Week 8						
n	29	29	12	12		
Mean (SD)	79.08 (15.090)	8.97 (15.663)	71.67 (22.940)	7.22 (20.392)		
Median	80.00	6.67	73.33	3.33		
Min, Max	33.3, 100.0	-26.7, 46.7	20.0, 100.0	-26.7, 46.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Physical Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	32	32	15	15		
Mean (SD)	83.54 (15.144)	12.50 (15.027)	67.11 (22.178)	2.67 (19.648)		
Median	86.67	6.67	73.33	0.00		
Min, Max	26.7, 100.0	-6.7, 46.7	20.0, 100.0	-33.3, 46.7		
Week 24						
n	29	29	14	14		
Mean (SD)	85.98 (13.492)	14.25 (18.493)	79.05 (25.062)	11.90 (11.746)		
Median	86.67	6.67	90.00	13.33		
Min, Max	40.0, 100.0	-6.7, 60.0	26.7, 100.0	-6.7, 33.3		

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Modified Randomized Set

Physical Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	12	12	41	41
Mean (SD)	81.84 (18.074)	10.11 (17.694)	75.56 (24.177)	5.00 (15.341)	80.00 (19.944)	8.62 (17.013)
Median	86.67	6.67	80.00	3.33	86.67	6.67
Min, Max	13.3, 100.0	-26.7, 53.3	20.0, 100.0	-26.7, 26.7	13.3, 100.0	-26.7, 53.3
Week 56						
n	21	21	10	10	31	31
Mean (SD)	81.90 (16.075)	9.21 (17.317)	81.33 (17.722)	7.33 (11.946)	81.72 (16.327)	8.60 (15.606)
Median	86.67	6.67	80.00	6.67	80.00	6.67
Min, Max	46.7, 100.0	-20.0, 60.0	53.3, 100.0	-13.3, 26.7	46.7, 100.0	-20.0, 60.0

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Physical Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	7	7	19	19
Mean (SD)	82.78 (12.858)	12.78 (18.303)	72.38 (31.840)	2.86 (21.725)	78.95 (21.575)	9.12 (19.653)
Median	83.33	10.00	86.67	6.67	86.67	6.67
Min, Max	60.0, 100.0	-13.3, 60.0	6.7, 100.0	-40.0, 20.0	6.7, 100.0	-40.0, 60.0
Week 88						
n	8	8	5	5	13	13
Mean (SD)	87.50 (13.063)	10.83 (11.785)	70.67 (26.916)	1.33 (12.824)	81.03 (20.339)	7.18 (12.609)
Median	90.00	6.67	66.67	0.00	86.67	6.67
Min, Max	60.0, 100.0	-6.7, 33.3	33.3, 100.0	-13.3, 20.0	33.3, 100.0	-13.3, 33.3

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Modified Randomized Set

Physical Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	96.67 (4.714)	10.00 (4.714)	93.33 (NA)	6.67 (NA)	95.56 (3.849)	8.89 (3.849)
Median	96.67	10.00	93.33	6.67	93.33	6.67
Min, Max	93.3, 100.0	6.7, 13.3	93.3, 93.3	6.7, 6.7	93.3, 100.0	6.7, 13.3

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Modified Randomized Set

Role Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		15		50	
Mean (SD)	66.67 (26.507)		63.33 (26.125)		65.67 (26.171)	
Median	66.67		66.67		66.67	
Min, Max	0.0, 100.0		16.7, 100.0		0.0, 100.0	
Week 2						
n	33	33	14	14		
Mean (SD)	73.74 (27.332)	8.08 (22.096)	72.62 (24.114)	7.14 (16.939)		
Median	83.33	0.00	66.67	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	16.7, 100.0	-33.3, 33.3		

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Modified Randomized Set

Role Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	35	35	14	14		
Mean (SD)	82.38 (20.589)	15.71 (22.846)	69.05 (26.840)	3.57 (30.786)		
Median	83.33	16.67	75.00	0.00		
Min, Max	0.0, 100.0	-16.7, 83.3	16.7, 100.0	-33.3, 83.3		
Week 8						
n	29	29	11	11		
Mean (SD)	76.44 (21.601)	10.34 (19.626)	74.24 (25.126)	6.06 (28.159)		
Median	83.33	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 50.0	33.3, 100.0	-33.3, 66.7		

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Modified Randomized Set

Role Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	32	32	14	14		
Mean (SD)	81.77 (22.938)	14.06 (22.042)	72.62 (28.947)	7.14 (34.415)		
Median	83.33	16.67	83.33	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	16.7, 100.0	-33.3, 66.7		
Week 24						
n	29	29	14	14		
Mean (SD)	80.46 (24.426)	12.07 (27.057)	80.95 (29.855)	15.48 (31.666)		
Median	83.33	0.00	91.67	25.00		
Min, Max	0.0, 100.0	-33.3, 83.3	0.0, 100.0	-33.3, 66.7		

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Role Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	12	12	41	41
Mean (SD)	78.16 (25.628)	7.47 (23.816)	72.22 (36.469)	9.72 (26.071)	76.42 (28.862)	8.13 (24.187)
Median	83.33	0.00	83.33	8.33	83.33	0.00
Min, Max	0.0, 100.0	-50.0, 66.7	0.0, 100.0	-33.3, 50.0	0.0, 100.0	-50.0, 66.7
Week 56						
n	21	21	10	10	31	31
Mean (SD)	79.37 (21.670)	7.14 (20.119)	88.33 (13.721)	23.33 (21.082)	82.26 (19.690)	12.37 (21.503)
Median	66.67	0.00	91.67	25.00	83.33	0.00
Min, Max	16.7, 100.0	-33.3, 66.7	66.7, 100.0	0.0, 66.7	16.7, 100.0	-33.3, 66.7

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Role Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	6	6	18	18
Mean (SD)	80.56 (22.285)	13.89 (28.278)	80.56 (24.533)	19.44 (19.484)	80.56 (22.324)	15.74 (25.226)
Median	83.33	8.33	91.67	25.00	91.67	16.67
Min, Max	33.3, 100.0	-33.3, 66.7	50.0, 100.0	-16.7, 33.3	33.3, 100.0	-33.3, 66.7
Week 88						
n	8	8	5	5	13	13
Mean (SD)	89.58 (15.269)	12.50 (19.416)	63.33 (32.059)	0.00 (20.412)	79.49 (25.598)	7.69 (19.971)
Median	100.00	0.00	66.67	0.00	83.33	0.00
Min, Max	66.7, 100.0	0.0, 50.0	16.7, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 50.0

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.11.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Role Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	100.00 (0.000)	0.00 (0.000)	100.00 (NA)	16.67 (NA)	100.00 (0.000)	5.56 (9.623)
Median	100.00	0.00	100.00	16.67	100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0	100.0, 100.0	16.7, 16.7	100.0, 100.0	0.0, 16.7

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.11.6

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Emotional Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		15		50	
Mean (SD)	80.24 (21.110)		77.22 (13.897)		79.33 (19.140)	
Median	83.33		75.00		83.33	
Min, Max	0.0, 100.0		50.0, 100.0		0.0, 100.0	
Week 2						
n	34	34	15	15		
Mean (SD)	85.78 (16.607)	5.64 (12.600)	76.67 (14.840)	-0.56 (15.580)		
Median	91.67	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-16.7, 33.3	33.3, 91.7	-33.3, 25.0		

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Modified Randomized Set

Emotional Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	35	35	15	15		
Mean (SD)	86.67 (15.016)	6.43 (14.726)	75.56 (17.947)	-1.67 (17.873)		
Median	91.67	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-33.3, 41.7	33.3, 100.0	-33.3, 33.3		
Week 8						
n	29	29	12	12		
Mean (SD)	83.33 (15.749)	3.74 (13.654)	85.42 (13.355)	5.56 (14.361)		
Median	83.33	0.00	83.33	0.00		
Min, Max	50.0, 100.0	-25.0, 33.3	66.7, 100.0	-16.7, 33.3		

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Modified Randomized Set

Emotional Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	32	32	15	15		
Mean (SD)	87.24 (18.692)	6.51 (11.147)	75.00 (16.366)	-2.22 (20.282)		
Median	100.00	4.17	75.00	0.00		
Min, Max	16.7, 100.0	-16.7, 33.3	50.0, 100.0	-50.0, 33.3		
Week 24						
n	29	29	14	14		
Mean (SD)	87.07 (19.993)	6.90 (12.213)	81.55 (14.683)	3.57 (18.407)		
Median	91.67	8.33	83.33	4.17		
Min, Max	8.3, 100.0	-16.7, 33.3	41.7, 100.0	-33.3, 33.3		

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Modified Randomized Set

Emotional Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	12	12	41	41
Mean (SD)	88.22 (18.300)	7.76 (13.535)	80.56 (14.794)	3.47 (16.460)	85.98 (17.524)	6.50 (14.375)
Median	100.00	8.33	75.00	0.00	91.67	0.00
Min, Max	16.7, 100.0	-33.3, 33.3	66.7, 100.0	-25.0, 33.3	16.7, 100.0	-33.3, 33.3
Week 56						
n	21	21	10	10	31	31
Mean (SD)	84.52 (20.119)	5.95 (17.309)	75.83 (15.441)	1.67 (20.337)	81.72 (18.932)	4.57 (18.110)
Median	91.67	0.00	79.17	0.00	83.33	0.00
Min, Max	33.3, 100.0	-41.7, 33.3	50.0, 100.0	-16.7, 41.7	33.3, 100.0	-41.7, 41.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Emotional Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	7	7	19	19
Mean (SD)	93.06 (11.143)	8.33 (18.803)	78.57 (12.599)	2.38 (17.817)	87.72 (13.428)	6.14 (18.182)
Median	100.00	4.17	75.00	0.00	91.67	0.00
Min, Max	66.7, 100.0	-33.3, 33.3	66.7, 100.0	-25.0, 33.3	66.7, 100.0	-33.3, 33.3
Week 88						
n	8	8	5	5	13	13
Mean (SD)	92.71 (8.259)	6.25 (10.681)	78.33 (20.069)	1.67 (27.889)	87.18 (15.068)	4.49 (18.199)
Median	91.67	4.17	83.33	0.00	91.67	0.00
Min, Max	75.0, 100.0	-8.3, 25.0	50.0, 100.0	-33.3, 33.3	50.0, 100.0	-33.3, 33.3

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Modified Randomized Set

Emotional Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	100.00 (0.000)	0.00 (0.000)	33.33 (NA)	-33.33 (NA)	77.78 (38.490)	-11.11 (19.245)
Median	100.00	0.00	33.33	-33.33	100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0	33.3, 33.3	-33.3, -33.3	33.3, 100.0	-33.3, 0.0

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Modified Randomized Set

Cognitive Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		15		50	
Mean (SD)	78.57 (23.421)		68.89 (24.289)		75.67 (23.859)	
Median	83.33		66.67		83.33	
Min, Max	16.7, 100.0		16.7, 100.0		16.7, 100.0	
Week 2						
n	34	34	15	15		
Mean (SD)	82.84 (17.641)	4.41 (18.491)	71.11 (29.187)	2.22 (25.092)		
Median	83.33	0.00	83.33	0.00		
Min, Max	50.0, 100.0	-16.7, 66.7	0.0, 100.0	-33.3, 66.7		

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Modified Randomized Set

Cognitive Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	35	35	15	15		
Mean (SD)	85.71 (15.730)	7.14 (16.313)	70.00 (30.342)	1.11 (26.327)		
Median	83.33	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-16.7, 50.0	0.0, 100.0	-33.3, 50.0		
Week 8						
n	29	29	12	12		
Mean (SD)	83.91 (17.527)	6.90 (16.377)	81.94 (22.983)	12.50 (21.468)		
Median	83.33	0.00	91.67	0.00		
Min, Max	33.3, 100.0	-33.3, 33.3	33.3, 100.0	-16.7, 50.0		

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Modified Randomized Set

Cognitive Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	32	32	15	15		
Mean (SD)	83.85 (18.686)	6.25 (15.698)	78.89 (26.327)	10.00 (22.537)		
Median	83.33	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-16.7, 50.0	16.7, 100.0	-16.7, 50.0		
Week 24						
n	29	29	14	14		
Mean (SD)	82.18 (23.959)	5.17 (16.728)	79.76 (24.615)	9.52 (14.194)		
Median	83.33	0.00	83.33	8.33		
Min, Max	0.0, 100.0	-16.7, 50.0	33.3, 100.0	-16.7, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Cognitive Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	12	12	41	41
Mean (SD)	85.06 (19.078)	8.62 (17.032)	70.83 (31.079)	-2.78 (17.164)	80.89 (23.735)	5.28 (17.654)
Median	100.00	0.00	75.00	0.00	83.33	0.00
Min, Max	33.3, 100.0	-33.3, 50.0	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 50.0
Week 56						
n	21	21	10	10	31	31
Mean (SD)	84.92 (20.348)	6.35 (15.344)	76.67 (31.623)	1.67 (24.152)	82.26 (24.319)	4.84 (18.355)
Median	100.00	0.00	83.33	8.33	83.33	0.00
Min, Max	33.3, 100.0	-16.7, 50.0	0.0, 100.0	-50.0, 33.3	0.0, 100.0	-50.0, 50.0

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Modified Randomized Set

Cognitive Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	6	6	18	18
Mean (SD)	91.67 (13.295)	5.56 (17.885)	75.00 (31.180)	5.56 (20.184)	86.11 (21.580)	5.56 (18.078)
Median	100.00	0.00	83.33	8.33	100.00	0.00
Min, Max	66.7, 100.0	-16.7, 50.0	16.7, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 50.0
Week 88						
n	8	8	5	5	13	13
Mean (SD)	97.92 (5.893)	6.25 (12.400)	56.67 (40.139)	-6.67 (27.889)	82.05 (31.521)	1.28 (19.792)
Median	100.00	0.00	66.67	0.00	100.00	0.00
Min, Max	83.3, 100.0	0.0, 33.3	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 33.3

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Modified Randomized Set

Cognitive Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	100.00 (0.000)	0.00 (0.000)	33.33 (NA)	-33.33 (NA)	77.78 (38.490)	-11.11 (19.245)
Median	100.00	0.00	33.33	-33.33	100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0	33.3, 33.3	-33.3, -33.3	33.3, 100.0	-33.3, 0.0

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Social Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	35		15		50	
Mean (SD)	71.90 (26.436)		68.89 (28.776)		71.00 (26.897)	
Median	66.67		66.67		66.67	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	35	35	14	14		
Mean (SD)	80.48 (20.407)	8.57 (25.364)	76.19 (24.209)	9.52 (19.298)		
Median	83.33	0.00	75.00	0.00		
Min, Max	33.3, 100.0	-50.0, 83.3	16.7, 100.0	-16.7, 50.0		

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Modified Randomized Set

Social Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	35	35	14	14		
Mean (SD)	83.81 (16.903)	11.90 (21.982)	73.81 (28.280)	7.14 (19.298)		
Median	83.33	0.00	75.00	0.00		
Min, Max	50.0, 100.0	-16.7, 83.3	0.0, 100.0	-16.7, 50.0		
Week 8						
n	29	29	11	11		
Mean (SD)	82.18 (21.330)	7.47 (19.203)	84.85 (17.408)	10.61 (18.668)		
Median	83.33	0.00	100.00	16.67		
Min, Max	33.3, 100.0	-50.0, 50.0	66.7, 100.0	-16.7, 33.3		

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Modified Randomized Set

Social Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	32	32	15	15		
Mean (SD)	82.81 (21.371)	10.94 (23.035)	67.78 (32.408)	-1.11 (27.070)		
Median	91.67	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 83.3	0.0, 100.0	-83.3, 33.3		
Week 24						
n	29	29	13	13		
Mean (SD)	82.18 (24.369)	10.34 (27.246)	79.49 (27.347)	15.38 (18.586)		
Median	100.00	0.00	83.33	16.67		
Min, Max	0.0, 100.0	-33.3, 83.3	0.0, 100.0	-16.7, 50.0		

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Social Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	12	12	41	41
Mean (SD)	75.29 (26.956)	1.15 (29.858)	70.83 (35.622)	4.17 (36.324)	73.98 (29.356)	2.03 (31.446)
Median	83.33	0.00	83.33	16.67	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 83.3	0.0, 100.0	-100.0, 33.3	0.0, 100.0	-100.0, 83.3
Week 56						
n	21	21	10	10	31	31
Mean (SD)	80.95 (21.913)	7.94 (28.195)	80.00 (21.943)	15.00 (19.954)	80.65 (21.558)	10.22 (25.704)
Median	83.33	0.00	83.33	16.67	83.33	0.00
Min, Max	33.3, 100.0	-50.0, 83.3	33.3, 100.0	-16.7, 50.0	33.3, 100.0	-50.0, 83.3

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Social Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	12	12	7	7	19	19
Mean (SD)	83.33 (23.570)	19.44 (28.278)	73.81 (28.637)	2.38 (39.002)	79.82 (25.202)	13.16 (32.669)
Median	100.00	8.33	83.33	16.67	83.33	16.67
Min, Max	33.3, 100.0	-16.7, 83.3	16.7, 100.0	-83.3, 33.3	16.7, 100.0	-83.3, 83.3
Week 88						
n	8	8	5	5	13	13
Mean (SD)	83.33 (17.817)	14.58 (28.781)	80.00 (18.257)	6.67 (25.276)	82.05 (17.296)	11.54 (26.688)
Median	83.33	0.00	66.67	16.67	66.67	0.00
Min, Max	66.7, 100.0	0.0, 83.3	66.7, 100.0	-33.3, 33.3	66.7, 100.0	-33.3, 83.3

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Modified Randomized Set

Social Functioning

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	100.00 (0.000)	16.67 (23.570)	100.00 (NA)	50.00 (NA)	100.00 (0.000)	27.78 (25.459)
Median	100.00	16.67	100.00	50.00	100.00	33.33
Min, Max	100.0, 100.0	0.0, 33.3	100.0, 100.0	50.0, 50.0	100.0, 100.0	0.0, 50.0

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Modified Randomized Set

Global Health Status/QoL

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	61.36 (20.175)		51.85 (14.894)		58.60 (19.062)	
Median	66.67		50.00		66.67	
Min, Max	33.3, 100.0		33.3, 66.7		33.3, 100.0	
Week 2						
n	22	22	10	8		
Mean (SD)	64.39 (22.593)	3.03 (17.733)	56.67 (19.954)	5.21 (17.783)		
Median	66.67	0.00	58.33	0.00		
Min, Max	16.7, 100.0	-50.0, 33.3	25.0, 83.3	-16.7, 33.3		

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Modified Randomized Set

Global Health Status/QoL

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	9		
Mean (SD)	67.42 (22.845)	6.06 (15.469)	56.67 (20.713)	1.85 (10.015)		
Median	75.00	0.00	62.50	0.00		
Min, Max	25.0, 100.0	-16.7, 41.7	25.0, 83.3	-8.3, 16.7		
Week 8						
n	16	16	7	6		
Mean (SD)	77.60 (15.729)	18.75 (16.805)	58.33 (24.056)	5.56 (17.213)		
Median	83.33	16.67	66.67	0.00		
Min, Max	41.7, 100.0	-16.7, 50.0	16.7, 83.3	-16.7, 33.3		

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Modified Randomized Set

Global Health Status/QoL

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	9		
Mean (SD)	75.00 (20.233)	13.33 (14.408)	63.33 (15.811)	10.19 (14.894)		
Median	75.00	16.67	66.67	8.33		
Min, Max	33.3, 100.0	-16.7, 33.3	33.3, 83.3	-8.3, 41.7		
Week 24						
n	20	20	10	9		
Mean (SD)	70.83 (17.832)	9.17 (13.218)	64.17 (12.454)	12.04 (16.724)		
Median	66.67	8.33	66.67	16.67		
Min, Max	33.3, 100.0	-16.7, 33.3	33.3, 83.3	-8.3, 33.3		

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Modified Randomized Set

Global Health Status/QoL

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	9	9	26	26
Mean (SD)	73.04 (18.049)	11.27 (14.416)	63.89 (16.137)	12.04 (16.197)	69.87 (17.650)	11.54 (14.734)
Median	66.67	0.00	66.67	16.67	66.67	12.50
Min, Max	50.0, 100.0	-8.3, 33.3	33.3, 83.3	-16.7, 33.3	33.3, 100.0	-16.7, 33.3
Week 56						
n	13	13	7	6	20	19
Mean (SD)	65.38 (21.204)	3.85 (16.179)	58.33 (25.909)	-2.78 (23.960)	62.92 (22.536)	1.75 (18.547)
Median	66.67	0.00	66.67	-8.33	66.67	0.00
Min, Max	33.3, 100.0	-16.7, 41.7	8.3, 83.3	-25.0, 41.7	8.3, 100.0	-25.0, 41.7

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Global Health Status/QoL

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	5	4	13	12
Mean (SD)	73.96 (15.064)	6.25 (27.728)	68.33 (19.003)	10.42 (32.896)	71.79 (16.151)	7.64 (28.082)
Median	70.83	4.17	66.67	4.17	66.67	4.17
Min, Max	50.0, 100.0	-50.0, 41.7	50.0, 91.7	-16.7, 50.0	50.0, 100.0	-50.0, 50.0
Week 88						
n	6	6	3	3	9	9
Mean (SD)	76.39 (15.290)	8.33 (17.480)	61.11 (19.245)	13.89 (29.266)	71.30 (17.236)	10.19 (20.318)
Median	75.00	8.33	50.00	16.67	66.67	16.67
Min, Max	58.3, 100.0	-16.7, 33.3	50.0, 83.3	-16.7, 41.7	50.0, 100.0	-16.7, 41.7

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Global Health Status/QoL

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	79.17 (29.463)	0.00 (0.000)	83.33 (NA)	41.67 (NA)	80.56 (20.972)	13.89 (24.056)
Median	79.17	0.00	83.33	41.67	83.33	0.00
Min, Max	58.3, 100.0	0.0, 0.0	83.3, 83.3	41.7, 41.7	58.3, 100.0	0.0, 41.7

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Physical Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	78.48 (17.690)		66.67 (15.275)		75.05 (17.636)	
Median	83.33		66.67		80.00	
Min, Max	40.0, 100.0		40.0, 86.7		40.0, 100.0	
Week 2						
n	22	22	10	8		
Mean (SD)	82.42 (19.899)	3.94 (9.575)	62.67 (17.554)	-5.83 (7.507)		
Median	86.67	0.00	66.67	-6.67		
Min, Max	40.0, 100.0	-13.3, 26.7	26.7, 80.0	-13.3, 6.7		

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Modified Randomized Set

Physical Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	9		
Mean (SD)	86.67 (17.457)	8.18 (11.626)	64.00 (23.349)	-5.19 (15.194)		
Median	93.33	6.67	66.67	-6.67		
Min, Max	40.0, 100.0	-20.0, 26.7	26.7, 100.0	-26.7, 13.3		
Week 8						
n	16	16	7	6		
Mean (SD)	91.67 (13.444)	11.25 (13.271)	68.57 (18.344)	-1.11 (5.018)		
Median	100.00	13.33	73.33	0.00		
Min, Max	53.3, 100.0	-13.3, 40.0	33.3, 86.7	-6.7, 6.7		

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Modified Randomized Set

Physical Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	9		
Mean (SD)	87.67 (15.488)	9.00 (10.874)	64.67 (18.606)	-3.70 (13.789)		
Median	93.33	6.67	66.67	0.00		
Min, Max	53.3, 100.0	-6.7, 33.3	33.3, 93.3	-33.3, 6.7		
Week 24						
n	20	20	10	9		
Mean (SD)	85.67 (16.931)	7.00 (13.241)	76.00 (18.379)	8.15 (15.556)		
Median	93.33	13.33	80.00	6.67		
Min, Max	53.3, 100.0	-33.3, 26.7	46.7, 100.0	-6.7, 40.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Physical Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	9	9	26	26
Mean (SD)	87.06 (16.578)	7.84 (9.785)	71.11 (26.874)	4.44 (14.142)	81.54 (21.607)	6.67 (11.314)
Median	93.33	6.67	73.33	6.67	86.67	6.67
Min, Max	53.3, 100.0	-13.3, 20.0	33.3, 100.0	-20.0, 26.7	33.3, 100.0	-20.0, 26.7
Week 56						
n	13	13	7	6	20	19
Mean (SD)	85.64 (16.963)	9.23 (10.377)	76.19 (17.578)	4.44 (19.626)	82.33 (17.341)	7.72 (13.565)
Median	86.67	13.33	80.00	6.67	86.67	13.33
Min, Max	53.3, 100.0	-6.7, 20.0	46.7, 100.0	-20.0, 33.3	46.7, 100.0	-20.0, 33.3

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.11.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Physical Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	5	4	14	13
Mean (SD)	86.67 (15.635)	6.67 (14.530)	78.67 (13.663)	11.67 (8.389)	83.81 (14.956)	8.21 (12.810)
Median	93.33	13.33	73.33	13.33	90.00	13.33
Min, Max	53.3, 100.0	-13.3, 26.7	66.7, 93.3	0.0, 20.0	53.3, 100.0	-13.3, 26.7
Week 88						
n	6	6	3	3	9	9
Mean (SD)	88.89 (13.771)	11.11 (10.887)	80.00 (24.037)	17.78 (21.430)	85.93 (16.814)	13.33 (14.142)
Median	93.33	10.00	86.67	26.67	86.67	13.33
Min, Max	66.7, 100.0	0.0, 26.7	53.3, 100.0	-6.7, 33.3	53.3, 100.0	-6.7, 33.3

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Modified Randomized Set

Physical Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	86.67 (18.856)	6.67 (9.428)	86.67 (NA)	33.33 (NA)	86.67 (13.333)	15.56 (16.777)
Median	86.67	6.67	86.67	33.33	86.67	13.33
Min, Max	73.3, 100.0	0.0, 13.3	86.7, 86.7	33.3, 33.3	73.3, 100.0	0.0, 33.3

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Modified Randomized Set

Role Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	68.18 (29.506)		62.96 (33.101)		66.67 (30.123)	
Median	66.67		66.67		66.67	
Min, Max	0.0, 100.0		16.7, 100.0		0.0, 100.0	
Week 2						
n	22	22	10	8		
Mean (SD)	77.27 (30.231)	9.09 (26.089)	61.67 (32.442)	-6.25 (15.269)		
Median	100.00	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-50.0, 66.7	0.0, 100.0	-33.3, 16.7		

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Role Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	9		
Mean (SD)	79.55 (29.065)	11.36 (33.879)	66.67 (27.217)	0.00 (16.667)		
Median	100.00	0.00	66.67	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	16.7, 100.0	-33.3, 16.7		
Week 8						
n	16	16	7	6		
Mean (SD)	90.63 (16.066)	22.92 (28.464)	57.14 (26.972)	-19.44 (24.533)		
Median	100.00	25.00	66.67	-16.67		
Min, Max	50.0, 100.0	-33.3, 83.3	16.7, 100.0	-66.7, 0.0		

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Modified Randomized Set

Role Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	9		
Mean (SD)	82.50 (21.948)	15.00 (28.045)	63.33 (29.187)	0.00 (35.355)		
Median	100.00	8.33	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 66.7	0.0, 100.0	-66.7, 50.0		
Week 24						
n	20	20	10	9		
Mean (SD)	73.33 (33.942)	5.83 (33.454)	65.00 (28.814)	1.85 (45.219)		
Median	91.67	0.00	66.67	16.67		
Min, Max	0.0, 100.0	-66.7, 50.0	0.0, 100.0	-100.0, 50.0		

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Role Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	9	9	26	26
Mean (SD)	83.33 (23.570)	14.71 (29.978)	62.96 (33.101)	0.00 (26.352)	76.28 (28.352)	9.62 (29.125)
Median	100.00	16.67	66.67	0.00	83.33	0.00
Min, Max	16.7, 100.0	-66.7, 66.7	16.7, 100.0	-50.0, 33.3	16.7, 100.0	-66.7, 66.7
Week 56						
n	13	13	7	6	20	19
Mean (SD)	66.67 (31.180)	-1.28 (36.932)	71.43 (12.599)	5.56 (34.427)	68.33 (25.876)	0.88 (35.344)
Median	66.67	0.00	66.67	8.33	66.67	0.00
Min, Max	0.0, 100.0	-83.3, 50.0	66.7, 100.0	-33.3, 50.0	0.0, 100.0	-83.3, 50.0

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Modified Randomized Set

Role Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	5	4	14	13
Mean (SD)	81.48 (34.805)	16.67 (23.570)	73.33 (19.003)	20.83 (36.956)	78.57 (29.547)	17.95 (26.755)
Median	100.00	16.67	66.67	16.67	91.67	16.67
Min, Max	0.0, 100.0	-16.7, 50.0	50.0, 100.0	-16.7, 66.7	0.0, 100.0	-16.7, 66.7
Week 88						
n	6	6	3	3	9	9
Mean (SD)	80.56 (30.581)	13.89 (24.533)	83.33 (16.667)	33.33 (33.333)	81.48 (25.610)	20.37 (27.358)
Median	100.00	8.33	83.33	33.33	100.00	16.67
Min, Max	33.3, 100.0	-16.7, 50.0	66.7, 100.0	0.0, 66.7	33.3, 100.0	-16.7, 66.7

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Modified Randomized Set

Role Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	75.00 (35.355)	8.33 (11.785)	66.67 (NA)	50.00 (NA)	72.22 (25.459)	22.22 (25.459)
Median	75.00	8.33	66.67	50.00	66.67	16.67
Min, Max	50.0, 100.0	0.0, 16.7	66.7, 66.7	50.0, 50.0	50.0, 100.0	0.0, 50.0

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Emotional Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	78.79 (24.764)		68.52 (36.271)		75.81 (28.330)	
Median	83.33		83.33		83.33	
Min, Max	0.0, 100.0		8.3, 100.0		0.0, 100.0	
Week 2						
n	22	22	10	8		
Mean (SD)	82.20 (20.621)	3.41 (19.528)	70.00 (28.653)	4.17 (14.773)		
Median	91.67	0.00	75.00	0.00		
Min, Max	41.7, 100.0	-25.0, 58.3	33.3, 100.0	-16.7, 25.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Emotional Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	9		
Mean (SD)	79.92 (30.717)	1.14 (24.165)	80.00 (28.109)	9.26 (12.805)		
Median	91.67	0.00	95.83	8.33		
Min, Max	0.0, 100.0	-66.7, 58.3	25.0, 100.0	-8.3, 33.3		
Week 8						
n	16	16	7	6		
Mean (SD)	88.54 (18.972)	11.98 (22.354)	79.76 (30.375)	5.56 (11.386)		
Median	100.00	8.33	91.67	8.33		
Min, Max	41.7, 100.0	-25.0, 58.3	16.7, 100.0	-8.3, 16.7		

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Modified Randomized Set

Emotional Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	9		
Mean (SD)	87.50 (18.829)	9.17 (18.117)	73.33 (33.054)	4.63 (13.889)		
Median	100.00	4.17	83.33	8.33		
Min, Max	41.7, 100.0	-8.3, 66.7	0.0, 100.0	-16.7, 25.0		
Week 24						
n	20	20	10	9		
Mean (SD)	81.67 (25.592)	3.33 (25.564)	80.00 (22.635)	12.04 (18.686)		
Median	91.67	0.00	83.33	8.33		
Min, Max	0.0, 100.0	-66.7, 66.7	33.3, 100.0	-16.7, 50.0		

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Emotional Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	9	9	26	26
Mean (SD)	80.39 (26.507)	-0.49 (25.425)	74.07 (23.365)	5.56 (27.951)	78.21 (25.175)	1.60 (25.929)
Median	91.67	0.00	75.00	8.33	87.50	0.00
Min, Max	0.0, 100.0	-66.7, 58.3	25.0, 100.0	-25.0, 66.7	0.0, 100.0	-66.7, 66.7
Week 56						
n	13	13	7	6	20	19
Mean (SD)	78.85 (29.583)	0.00 (28.260)	83.33 (22.048)	20.83 (31.513)	80.42 (26.666)	6.58 (30.121)
Median	91.67	0.00	91.67	16.67	91.67	0.00
Min, Max	0.0, 100.0	-66.7, 50.0	41.7, 100.0	-16.7, 75.0	0.0, 100.0	-66.7, 75.0

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Emotional Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	5	4	13	12
Mean (SD)	87.50 (16.060)	1.04 (10.387)	73.33 (36.987)	20.83 (8.333)	82.05 (25.650)	7.64 (13.511)
Median	95.83	0.00	100.00	16.67	100.00	8.33
Min, Max	58.3, 100.0	-16.7, 16.7	25.0, 100.0	16.7, 33.3	25.0, 100.0	-16.7, 33.3
Week 88						
n	6	6	3	3	9	9
Mean (SD)	88.89 (17.213)	6.94 (12.266)	75.00 (36.324)	41.67 (36.324)	84.26 (23.733)	18.52 (26.932)
Median	100.00	4.17	91.67	25.00	100.00	16.67
Min, Max	66.7, 100.0	-8.3, 25.0	33.3, 100.0	16.7, 83.3	33.3, 100.0	-8.3, 83.3

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Modified Randomized Set

Emotional Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	91.67 (11.785)	4.17 (5.893)	91.67 (NA)	83.33 (NA)	91.67 (8.333)	30.56 (45.896)
Median	91.67	4.17	91.67	83.33	91.67	8.33
Min, Max	83.3, 100.0	0.0, 8.3	91.7, 91.7	83.3, 83.3	83.3, 100.0	0.0, 83.3

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Cognitive Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	78.79 (21.932)		72.22 (31.180)		76.88 (24.600)	
Median	83.33		66.67		83.33	
Min, Max	16.7, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	22	22	9	8		
Mean (SD)	83.33 (19.245)	4.55 (14.712)	77.78 (26.352)	8.33 (23.570)		
Median	83.33	0.00	83.33	16.67		
Min, Max	33.3, 100.0	-16.7, 50.0	16.7, 100.0	-33.3, 33.3		

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Cognitive Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	9		
Mean (SD)	82.58 (20.878)	3.79 (21.780)	73.33 (30.631)	0.00 (23.570)		
Median	83.33	0.00	83.33	0.00		
Min, Max	16.7, 100.0	-50.0, 50.0	0.0, 100.0	-33.3, 33.3		
Week 8						
n	16	16	7	6		
Mean (SD)	84.38 (14.232)	9.38 (21.054)	69.05 (32.530)	2.78 (19.484)		
Median	83.33	0.00	83.33	8.33		
Min, Max	66.7, 100.0	-16.7, 66.7	0.0, 100.0	-33.3, 16.7		

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Cognitive Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	9		
Mean (SD)	86.67 (15.858)	8.33 (14.809)	70.00 (25.820)	-3.70 (21.695)		
Median	91.67	0.00	83.33	0.00		
Min, Max	50.0, 100.0	-16.7, 33.3	16.7, 100.0	-33.3, 16.7		
Week 24						
n	20	20	10	9		
Mean (SD)	82.50 (25.058)	4.17 (25.862)	76.67 (19.563)	3.70 (23.241)		
Median	91.67	0.00	83.33	0.00		
Min, Max	0.0, 100.0	-66.7, 50.0	33.3, 100.0	-33.3, 33.3		

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Modified Randomized Set

Cognitive Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	9	9	26	26
Mean (SD)	77.45 (26.965)	-0.98 (24.630)	70.37 (23.241)	-1.85 (22.737)	75.00 (25.495)	-1.28 (23.534)
Median	83.33	0.00	66.67	0.00	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	16.7, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 33.3
Week 56						
n	13	13	7	6	20	19
Mean (SD)	83.33 (25.459)	1.28 (24.964)	69.05 (32.530)	0.00 (18.257)	78.33 (28.150)	0.88 (22.549)
Median	100.00	0.00	83.33	0.00	83.33	0.00
Min, Max	16.7, 100.0	-50.0, 33.3	0.0, 100.0	-16.7, 33.3	0.0, 100.0	-50.0, 33.3

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Table GERMANY.2.5.11.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Cognitive Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	5	4	13	12
Mean (SD)	91.67 (12.599)	8.33 (12.599)	73.33 (34.561)	8.33 (9.623)	84.62 (24.019)	8.33 (11.237)
Median	100.00	0.00	83.33	8.33	100.00	0.00
Min, Max	66.7, 100.0	0.0, 33.3	16.7, 100.0	0.0, 16.7	16.7, 100.0	0.0, 33.3
Week 88						
n	6	6	3	3	9	9
Mean (SD)	88.89 (13.608)	8.33 (13.944)	66.67 (44.096)	11.11 (25.459)	81.48 (26.932)	9.26 (16.897)
Median	91.67	0.00	83.33	16.67	83.33	0.00
Min, Max	66.7, 100.0	0.0, 33.3	16.7, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 33.3

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Table GERMANY.2.5.11.6

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Modified Randomized Set

Cognitive Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	83.33 (23.570)	0.00 (0.000)	83.33 (NA)	16.67 (NA)	83.33 (16.667)	5.56 (9.623)
Median	83.33	0.00	83.33	16.67	83.33	0.00
Min, Max	66.7, 100.0	0.0, 0.0	83.3, 83.3	16.7, 16.7	66.7, 100.0	0.0, 16.7

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Table GERMANY.2.5.11.6

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and \geq 8.5 g/dL)
Modified Randomized Set

Social Functioning

Screening Hgb Level \geq 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	81.06 (24.286)		70.37 (37.060)		77.96 (28.345)	
Median	91.67		83.33		83.33	
Min, Max	16.7, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	22	22	10	8		
Mean (SD)	85.61 (22.000)	4.55 (16.412)	65.00 (33.747)	-10.42 (12.400)		
Median	100.00	0.00	66.67	-8.33		
Min, Max	33.3, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 0.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Social Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	9		
Mean (SD)	82.58 (26.466)	1.52 (18.480)	70.00 (26.989)	-3.70 (27.358)		
Median	100.00	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 33.3	16.7, 100.0	-50.0, 50.0		
Week 8						
n	16	16	7	6		
Mean (SD)	93.75 (11.980)	14.58 (27.806)	73.81 (28.637)	-2.78 (12.546)		
Median	100.00	8.33	83.33	0.00		
Min, Max	66.7, 100.0	-33.3, 83.3	16.7, 100.0	-16.7, 16.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Social Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	9		
Mean (SD)	92.50 (12.653)	10.00 (16.579)	68.33 (32.820)	-5.56 (26.352)		
Median	100.00	0.00	75.00	0.00		
Min, Max	66.7, 100.0	-16.7, 50.0	0.0, 100.0	-66.7, 33.3		
Week 24						
n	20	20	10	9		
Mean (SD)	81.67 (27.519)	-0.83 (23.242)	70.00 (23.307)	-3.70 (27.358)		
Median	100.00	0.00	66.67	-16.67		
Min, Max	0.0, 100.0	-66.7, 33.3	16.7, 100.0	-33.3, 50.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Social Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	9	9	26	26
Mean (SD)	88.24 (18.413)	4.90 (22.642)	48.15 (33.793)	-22.22 (53.359)	74.36 (30.995)	-4.49 (37.582)
Median	100.00	0.00	50.00	0.00	83.33	0.00
Min, Max	50.0, 100.0	-50.0, 50.0	0.0, 83.3	-100.0, 50.0	0.0, 100.0	-100.0, 50.0
Week 56						
n	13	13	7	6	20	19
Mean (SD)	88.46 (20.844)	7.69 (19.971)	73.81 (16.265)	-2.78 (38.610)	83.33 (20.233)	4.39 (26.552)
Median	100.00	0.00	66.67	-8.33	91.67	0.00
Min, Max	33.3, 100.0	-33.3, 33.3	50.0, 100.0	-50.0, 66.7	33.3, 100.0	-50.0, 66.7

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Social Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	4	3	12	11
Mean (SD)	95.83 (7.715)	12.50 (21.362)	91.67 (16.667)	0.00 (16.667)	94.44 (10.856)	9.09 (20.226)
Median	100.00	8.33	100.00	0.00	100.00	0.00
Min, Max	83.3, 100.0	-16.7, 50.0	66.7, 100.0	-16.7, 16.7	66.7, 100.0	-16.7, 50.0
Week 88						
n	6	6	3	3	9	9
Mean (SD)	91.67 (13.944)	8.33 (17.480)	77.78 (19.245)	16.67 (50.000)	87.04 (16.197)	11.11 (28.868)
Median	100.00	8.33	66.67	16.67	100.00	16.67
Min, Max	66.7, 100.0	-16.7, 33.3	66.7, 100.0	-33.3, 66.7	66.7, 100.0	-33.3, 66.7

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Modified Randomized Set

Social Functioning

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	1	1	3	3
Mean (SD)	83.33 (23.570)	-16.67 (23.570)	66.67 (NA)	66.67 (NA)	77.78 (19.245)	11.11 (50.918)
Median	83.33	-16.67	66.67	66.67	66.67	0.00
Min, Max	66.7, 100.0	-33.3, 0.0	66.7, 66.7	66.7, 66.7	66.7, 100.0	-33.3, 66.7

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Table GERMANY.2.5.11.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Global Health Status/QoL

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		10		31	
Mean (SD)	58.73 (20.323)		59.17 (12.076)		58.87 (17.865)	
Median	66.67		62.50		66.67	
Min, Max	16.7, 91.7		33.3, 75.0		16.7, 91.7	
Week 2						
n	21	21	11	9		
Mean (SD)	63.89 (19.954)	5.16 (16.557)	60.61 (13.484)	1.85 (14.299)		
Median	66.67	0.00	58.33	0.00		
Min, Max	33.3, 100.0	-41.7, 33.3	41.7, 83.3	-25.0, 25.0		

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Table GERMANY.2.5.11.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Global Health Status/QoL

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	21	21	11	10		
Mean (SD)	61.90 (18.924)	3.17 (13.037)	59.09 (20.566)	-2.50 (18.447)		
Median	66.67	0.00	58.33	0.00		
Min, Max	33.3, 91.7	-16.7, 33.3	33.3, 83.3	-41.7, 25.0		
Week 8						
n	14	14	6	5		
Mean (SD)	66.07 (24.122)	5.36 (23.481)	59.72 (24.954)	-6.67 (23.124)		
Median	66.67	4.17	62.50	0.00		
Min, Max	16.7, 100.0	-58.3, 33.3	16.7, 83.3	-33.3, 25.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Global Health Status/QoL

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	17	17	11	10		
Mean (SD)	68.14 (20.462)	11.76 (15.040)	58.33 (17.873)	-2.50 (17.146)		
Median	66.67	16.67	50.00	-4.17		
Min, Max	33.3, 100.0	-16.7, 33.3	33.3, 83.3	-25.0, 25.0		
Week 24						
n	14	14	11	10		
Mean (SD)	66.67 (23.342)	6.55 (16.072)	67.42 (17.660)	8.33 (15.215)		
Median	66.67	8.33	66.67	4.17		
Min, Max	16.7, 100.0	-33.3, 33.3	33.3, 100.0	-8.3, 41.7		

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Global Health Status/QoL

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	10	10	27	27
Mean (SD)	62.75 (21.065)	8.33 (19.320)	61.67 (23.307)	2.50 (15.738)	62.35 (21.480)	6.17 (17.991)
Median	66.67	8.33	66.67	0.00	66.67	0.00
Min, Max	33.3, 100.0	-41.7, 33.3	16.7, 100.0	-16.7, 25.0	16.7, 100.0	-41.7, 33.3
Week 56						
n	12	12	10	9	22	21
Mean (SD)	67.36 (18.620)	11.81 (19.286)	60.00 (16.102)	-2.78 (19.094)	64.02 (17.515)	5.56 (20.127)
Median	70.83	12.50	58.33	0.00	66.67	0.00
Min, Max	33.3, 91.7	-16.7, 41.7	33.3, 83.3	-25.0, 25.0	33.3, 91.7	-25.0, 41.7

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Global Health Status/QoL

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	7	7	8	7	15	14
Mean (SD)	71.43 (19.159)	11.90 (19.159)	66.67 (20.412)	0.00 (17.347)	68.89 (19.279)	5.95 (18.613)
Median	75.00	16.67	70.83	0.00	75.00	4.17
Min, Max	33.3, 91.7	-16.7, 41.7	33.3, 91.7	-16.7, 25.0	33.3, 91.7	-16.7, 41.7
Week 88						
n	5	5	5	5	10	10
Mean (SD)	75.00 (11.785)	11.67 (20.069)	58.33 (26.352)	-5.00 (22.515)	66.67 (21.155)	3.33 (21.943)
Median	66.67	16.67	66.67	-8.33	66.67	4.17
Min, Max	66.7, 91.7	-16.7, 33.3	16.7, 83.3	-33.3, 25.0	16.7, 91.7	-33.3, 33.3

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Global Health Status/QoL

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	100.00 (NA)	33.33 (NA)	75.00 (NA)	8.33 (NA)	87.50 (17.678)	20.83 (17.678)
Median	100.00	33.33	75.00	8.33	87.50	20.83
Min, Max	100.0, 100.0	33.3, 33.3	75.0, 75.0	8.3, 8.3	75.0, 100.0	8.3, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Physical Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		10		31	
Mean (SD)	70.16 (16.947)		67.33 (18.974)		69.25 (17.357)	
Median	66.67		70.00		66.67	
Min, Max	33.3, 100.0		26.7, 86.7		26.7, 100.0	
Week 2						
n	21	21	11	9		
Mean (SD)	73.97 (19.878)	3.81 (9.562)	69.09 (15.282)	1.48 (9.876)		
Median	80.00	0.00	73.33	0.00		
Min, Max	26.7, 100.0	-13.3, 26.7	46.7, 93.3	-13.3, 20.0		

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Physical Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	21	21	11	10		
Mean (SD)	78.73 (17.463)	8.57 (11.381)	63.03 (19.858)	-6.67 (12.958)		
Median	80.00	6.67	60.00	-3.33		
Min, Max	40.0, 100.0	-20.0, 26.7	33.3, 86.7	-26.7, 6.7		
Week 8						
n	14	14	6	5		
Mean (SD)	80.95 (16.508)	8.10 (9.846)	63.33 (27.244)	-8.00 (15.916)		
Median	83.33	10.00	66.67	-6.67		
Min, Max	53.3, 100.0	-13.3, 20.0	20.0, 93.3	-26.7, 13.3		

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Physical Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	17	17	11	10		
Mean (SD)	77.65 (19.712)	7.06 (9.271)	62.42 (19.612)	-6.67 (18.325)		
Median	80.00	6.67	66.67	0.00		
Min, Max	26.7, 100.0	-6.7, 20.0	20.0, 86.7	-33.3, 20.0		
Week 24						
n	14	14	11	10		
Mean (SD)	80.48 (18.576)	8.57 (9.933)	80.00 (21.292)	12.00 (10.796)		
Median	86.67	10.00	86.67	13.33		
Min, Max	40.0, 100.0	-6.7, 20.0	40.0, 100.0	-6.7, 33.3		

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Physical Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	10	10	27	27
Mean (SD)	75.69 (22.353)	7.06 (14.995)	74.00 (25.425)	6.67 (16.330)	75.06 (23.064)	6.91 (15.189)
Median	80.00	13.33	80.00	6.67	80.00	6.67
Min, Max	13.3, 100.0	-26.7, 26.7	20.0, 100.0	-26.7, 26.7	13.3, 100.0	-26.7, 26.7
Week 56						
n	12	12	10	9	22	21
Mean (SD)	77.22 (17.857)	10.00 (10.050)	74.67 (17.441)	4.44 (15.275)	76.06 (17.294)	7.62 (12.523)
Median	80.00	13.33	76.67	0.00	76.67	13.33
Min, Max	46.7, 100.0	-6.7, 20.0	46.7, 100.0	-20.0, 26.7	46.7, 100.0	-20.0, 26.7

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Physical Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	7	7	8	7	15	14
Mean (SD)	87.62 (10.491)	16.19 (8.483)	74.17 (29.694)	2.86 (20.676)	80.44 (23.158)	9.52 (16.685)
Median	86.67	13.33	80.00	6.67	86.67	13.33
Min, Max	73.3, 100.0	6.7, 26.7	6.7, 100.0	-40.0, 20.0	6.7, 100.0	-40.0, 26.7
Week 88						
n	5	5	5	5	10	10
Mean (SD)	90.67 (8.944)	16.00 (7.601)	68.00 (28.048)	0.00 (13.333)	79.33 (22.976)	8.00 (13.259)
Median	86.67	13.33	60.00	-6.67	86.67	10.00
Min, Max	80.0, 100.0	6.7, 26.7	33.3, 100.0	-13.3, 20.0	33.3, 100.0	-13.3, 26.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Physical Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	93.33 (NA)	13.33 (NA)	93.33 (NA)	6.67 (NA)	93.33 (0.000)	10.00 (4.714)
Median	93.33	13.33	93.33	6.67	93.33	10.00
Min, Max	93.3, 93.3	13.3, 13.3	93.3, 93.3	6.7, 6.7	93.3, 93.3	6.7, 13.3

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Role Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		10		31	
Mean (SD)	72.22 (21.943)		55.00 (26.117)		66.67 (24.343)	
Median	66.67		58.33		66.67	
Min, Max	16.7, 100.0		16.7, 100.0		16.7, 100.0	
Week 2						
n	21	21	10	8		
Mean (SD)	72.22 (30.882)	0.00 (22.361)	68.33 (25.398)	8.33 (12.599)		
Median	83.33	0.00	66.67	0.00		
Min, Max	0.0, 100.0	-50.0, 33.3	16.7, 100.0	0.0, 33.3		

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Role Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	21	21	10	9		
Mean (SD)	74.60 (27.698)	2.38 (19.920)	65.00 (27.722)	3.70 (35.136)		
Median	83.33	0.00	66.67	0.00		
Min, Max	0.0, 100.0	-50.0, 33.3	16.7, 100.0	-33.3, 83.3		
Week 8						
n	14	14	5	4		
Mean (SD)	78.57 (21.111)	2.38 (19.455)	63.33 (29.814)	0.00 (23.570)		
Median	83.33	0.00	66.67	8.33		
Min, Max	33.3, 100.0	-33.3, 33.3	33.3, 100.0	-33.3, 16.7		

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Role Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	17	17	10	9		
Mean (SD)	76.47 (28.903)	3.92 (20.858)	65.00 (29.866)	7.41 (42.583)		
Median	83.33	0.00	75.00	0.00		
Min, Max	0.0, 100.0	-33.3, 33.3	16.7, 100.0	-66.7, 66.7		
Week 24						
n	14	14	11	10		
Mean (SD)	69.05 (35.720)	-5.95 (24.114)	68.18 (36.098)	13.33 (48.305)		
Median	83.33	0.00	83.33	33.33		
Min, Max	0.0, 100.0	-50.0, 33.3	0.0, 100.0	-100.0, 66.7		

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Role Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	10	10	27	27
Mean (SD)	71.57 (32.684)	0.98 (27.304)	61.67 (42.346)	6.67 (36.175)	67.90 (36.081)	3.09 (30.325)
Median	66.67	0.00	75.00	25.00	66.67	0.00
Min, Max	0.0, 100.0	-66.7, 50.0	0.0, 100.0	-50.0, 50.0	0.0, 100.0	-66.7, 50.0
Week 56						
n	12	12	10	9	22	21
Mean (SD)	68.06 (35.146)	-4.17 (28.538)	78.33 (13.721)	22.22 (27.639)	72.73 (27.480)	7.14 (30.538)
Median	66.67	0.00	75.00	33.33	66.67	0.00
Min, Max	0.0, 100.0	-83.3, 33.3	66.7, 100.0	-33.3, 66.7	0.0, 100.0	-83.3, 66.7

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Role Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	7	7	7	6	14	13
Mean (SD)	80.95 (26.227)	7.14 (28.637)	80.95 (20.250)	27.78 (22.771)	80.95 (22.510)	16.67 (27.217)
Median	100.00	0.00	83.33	25.00	91.67	16.67
Min, Max	33.3, 100.0	-33.3, 50.0	50.0, 100.0	0.0, 66.7	33.3, 100.0	-33.3, 66.7
Week 88						
n	5	5	5	5	10	10
Mean (SD)	83.33 (28.868)	3.33 (18.257)	63.33 (32.059)	6.67 (25.276)	73.33 (30.631)	5.00 (20.861)
Median	100.00	0.00	66.67	0.00	83.33	0.00
Min, Max	33.3, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 33.3

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Role Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	100.00 (NA)	0.00 (NA)	100.00 (NA)	16.67 (NA)	100.00 (0.000)	8.33 (11.785)
Median	100.00	0.00	100.00	16.67	100.00	8.33
Min, Max	100.0, 100.0	0.0, 0.0	100.0, 100.0	16.7, 16.7	100.0, 100.0	0.0, 16.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Emotional Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		10		31	
Mean (SD)	80.95 (23.738)		78.33 (15.811)		80.11 (21.265)	
Median	83.33		83.33		83.33	
Min, Max	0.0, 100.0		50.0, 100.0		0.0, 100.0	
Week 2						
n	21	21	11	9		
Mean (SD)	85.32 (19.703)	4.37 (12.253)	74.24 (23.409)	-1.85 (18.530)		
Median	91.67	0.00	75.00	0.00		
Min, Max	33.3, 100.0	-16.7, 33.3	33.3, 100.0	-33.3, 25.0		

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Emotional Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	21	21	11	10		
Mean (SD)	81.35 (22.190)	0.40 (18.907)	80.30 (22.445)	0.00 (22.567)		
Median	91.67	0.00	83.33	0.00		
Min, Max	25.0, 100.0	-50.0, 33.3	33.3, 100.0	-33.3, 33.3		
Week 8						
n	14	14	6	5		
Mean (SD)	88.69 (14.838)	5.36 (11.136)	94.44 (8.607)	13.33 (18.257)		
Median	100.00	0.00	100.00	16.67		
Min, Max	58.3, 100.0	-8.3, 25.0	83.3, 100.0	-16.7, 33.3		

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Emotional Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	17	17	11	10		
Mean (SD)	85.29 (23.668)	4.41 (12.882)	75.76 (19.527)	-2.50 (24.548)		
Median	100.00	0.00	75.00	0.00		
Min, Max	16.7, 100.0	-16.7, 25.0	50.0, 100.0	-50.0, 33.3		
Week 24						
n	14	14	11	10		
Mean (SD)	83.33 (25.737)	3.57 (13.757)	88.64 (9.334)	11.67 (13.721)		
Median	91.67	0.00	91.67	8.33		
Min, Max	8.3, 100.0	-16.7, 25.0	75.0, 100.0	-8.3, 33.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Emotional Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	10	10	27	27
Mean (SD)	82.84 (21.342)	4.41 (16.169)	80.00 (14.803)	1.67 (19.563)	81.79 (18.924)	3.40 (17.181)
Median	91.67	0.00	75.00	0.00	83.33	0.00
Min, Max	16.7, 100.0	-33.3, 25.0	58.3, 100.0	-25.0, 33.3	16.7, 100.0	-33.3, 33.3
Week 56						
n	12	12	10	9	22	21
Mean (SD)	78.47 (24.989)	4.17 (22.613)	81.67 (17.916)	4.63 (21.695)	79.92 (21.618)	4.37 (21.670)
Median	91.67	4.17	87.50	0.00	91.67	0.00
Min, Max	33.3, 100.0	-41.7, 33.3	50.0, 100.0	-16.7, 41.7	33.3, 100.0	-41.7, 41.7

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Table GERMANY.2.5.11.7

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Emotional Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	7	7	8	7	15	14
Mean (SD)	91.67 (14.434)	5.95 (21.362)	87.50 (15.430)	7.14 (18.276)	89.44 (14.593)	6.55 (19.109)
Median	100.00	8.33	95.83	8.33	100.00	8.33
Min, Max	66.7, 100.0	-33.3, 33.3	66.7, 100.0	-25.0, 33.3	66.7, 100.0	-33.3, 33.3
Week 88						
n	5	5	5	5	10	10
Mean (SD)	86.67 (15.138)	6.67 (6.972)	80.00 (21.731)	0.00 (26.352)	83.33 (18.002)	3.33 (18.509)
Median	91.67	8.33	83.33	0.00	87.50	4.17
Min, Max	66.7, 100.0	0.0, 16.7	50.0, 100.0	-33.3, 33.3	50.0, 100.0	-33.3, 33.3

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Emotional Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	100.00 (NA)	0.00 (NA)	33.33 (NA)	-33.33 (NA)	66.67 (47.140)	-16.67 (23.570)
Median	100.00	0.00	33.33	-33.33	66.67	-16.67
Min, Max	100.0, 100.0	0.0, 0.0	33.3, 33.3	-33.3, -33.3	33.3, 100.0	-33.3, 0.0

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Cognitive Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		10		31	
Mean (SD)	80.95 (22.537)		71.67 (20.861)		77.96 (22.105)	
Median	83.33		66.67		83.33	
Min, Max	16.7, 100.0		33.3, 100.0		16.7, 100.0	
Week 2						
n	21	21	10	9		
Mean (SD)	85.71 (19.211)	4.76 (14.086)	68.33 (34.650)	-1.85 (25.610)		
Median	100.00	0.00	83.33	0.00		
Min, Max	50.0, 100.0	-16.7, 33.3	0.0, 100.0	-33.3, 33.3		

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Cognitive Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	21	21	11	10		
Mean (SD)	84.13 (18.616)	3.17 (15.473)	65.15 (32.876)	-8.33 (23.895)		
Median	83.33	0.00	66.67	-16.67		
Min, Max	33.3, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 33.3		
Week 8						
n	14	14	6	5		
Mean (SD)	89.29 (14.032)	9.52 (14.194)	80.56 (24.533)	16.67 (11.785)		
Median	100.00	8.33	83.33	16.67		
Min, Max	66.7, 100.0	-16.7, 33.3	33.3, 100.0	0.0, 33.3		

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Cognitive Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	17	17	11	10		
Mean (SD)	83.33 (22.822)	4.90 (12.862)	75.76 (27.247)	3.33 (20.488)		
Median	100.00	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 33.3		
Week 24						
n	14	14	11	10		
Mean (SD)	76.19 (31.834)	-1.19 (15.281)	77.27 (23.889)	5.00 (17.656)		
Median	91.67	0.00	83.33	8.33		
Min, Max	0.0, 100.0	-33.3, 33.3	33.3, 100.0	-16.7, 33.3		

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Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	10	10	27	27
Mean (SD)	78.43 (24.836)	1.96 (17.562)	66.67 (31.427)	-5.00 (22.292)	74.07 (27.477)	-0.62 (19.327)
Median	83.33	0.00	75.00	-8.33	83.33	0.00
Min, Max	33.3, 100.0	-50.0, 33.3	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-50.0, 33.3
Week 56						
n	12	12	10	9	22	21
Mean (SD)	77.78 (25.950)	2.78 (17.164)	76.67 (29.606)	0.00 (25.000)	77.27 (26.995)	1.59 (20.348)
Median	91.67	0.00	83.33	0.00	83.33	0.00
Min, Max	33.3, 100.0	-33.3, 33.3	0.0, 100.0	-50.0, 33.3	0.0, 100.0	-50.0, 33.3

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Cognitive Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	7	7	7	6	14	13
Mean (SD)	92.86 (13.113)	4.76 (15.853)	80.95 (31.074)	8.33 (17.480)	86.90 (23.732)	6.41 (16.013)
Median	100.00	0.00	100.00	8.33	100.00	0.00
Min, Max	66.7, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 33.3
Week 88						
n	5	5	5	5	10	10
Mean (SD)	93.33 (14.907)	6.67 (14.907)	60.00 (41.833)	-10.00 (27.889)	76.67 (34.427)	-1.67 (22.839)
Median	100.00	0.00	83.33	-16.67	91.67	0.00
Min, Max	66.7, 100.0	0.0, 33.3	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 33.3

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Cognitive Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	100.00 (NA)	0.00 (NA)	33.33 (NA)	-33.33 (NA)	66.67 (47.140)	-16.67 (23.570)
Median	100.00	0.00	33.33	-33.33	66.67	-16.67
Min, Max	100.0, 100.0	0.0, 0.0	33.3, 33.3	-33.3, -33.3	33.3, 100.0	-33.3, 0.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Social Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		10		31	
Mean (SD)	77.78 (23.174)		68.33 (30.882)		74.73 (25.774)	
Median	83.33		66.67		66.67	
Min, Max	33.3, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	21	21	10	8		
Mean (SD)	76.98 (22.033)	-0.79 (17.059)	73.33 (27.442)	6.25 (26.633)		
Median	83.33	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-50.0, 33.3	16.7, 100.0	-33.3, 50.0		

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Social Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	21	21	10	9		
Mean (SD)	79.37 (21.670)	1.59 (17.404)	63.33 (29.187)	-5.56 (23.570)		
Median	83.33	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 33.3	0.0, 100.0	-50.0, 33.3		
Week 8						
n	14	14	5	4		
Mean (SD)	83.33 (17.296)	1.19 (24.862)	93.33 (14.907)	25.00 (9.623)		
Median	83.33	0.00	100.00	25.00		
Min, Max	50.0, 100.0	-50.0, 50.0	66.7, 100.0	16.7, 33.3		

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Social Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	17	17	11	10		
Mean (SD)	81.37 (21.955)	2.94 (19.751)	60.61 (34.378)	-11.67 (36.047)		
Median	83.33	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 33.3	0.0, 100.0	-83.3, 33.3		
Week 24						
n	14	14	10	9		
Mean (SD)	75.00 (31.858)	-4.76 (17.817)	75.00 (29.659)	7.41 (25.154)		
Median	91.67	0.00	83.33	16.67		
Min, Max	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 33.3		

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Social Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	17	10	10	27	27
Mean (SD)	67.65 (30.317)	-6.86 (30.080)	50.00 (44.444)	-18.33 (57.440)	61.11 (36.398)	-11.11 (41.603)
Median	66.67	0.00	66.67	8.33	66.67	0.00
Min, Max	0.0, 100.0	-66.7, 50.0	0.0, 100.0	-100.0, 33.3	0.0, 100.0	-100.0, 50.0
Week 56						
n	12	12	10	9	22	21
Mean (SD)	76.39 (25.084)	4.17 (20.257)	75.00 (22.567)	7.41 (31.304)	75.76 (23.417)	5.56 (24.907)
Median	75.00	0.00	75.00	16.67	75.00	0.00
Min, Max	33.3, 100.0	-50.0, 33.3	33.3, 100.0	-50.0, 50.0	33.3, 100.0	-50.0, 50.0

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Social Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	7	7	8	7	15	14
Mean (SD)	83.33 (25.459)	16.67 (23.570)	75.00 (28.172)	-2.38 (39.002)	78.89 (26.327)	7.14 (32.499)
Median	100.00	16.67	75.00	16.67	83.33	16.67
Min, Max	33.3, 100.0	-16.7, 50.0	16.7, 100.0	-83.3, 33.3	16.7, 100.0	-83.3, 50.0
Week 88						
n	5	5	5	5	10	10
Mean (SD)	80.00 (18.257)	10.00 (14.907)	73.33 (14.907)	-3.33 (29.814)	76.67 (16.102)	3.33 (23.307)
Median	66.67	0.00	66.67	0.00	66.67	0.00
Min, Max	66.7, 100.0	0.0, 33.3	66.7, 100.0	-33.3, 33.3	66.7, 100.0	-33.3, 33.3

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Social Functioning

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	100.00 (NA)	33.33 (NA)	100.00 (NA)	50.00 (NA)	100.00 (0.000)	41.67 (11.785)
Median	100.00	33.33	100.00	50.00	100.00	41.67
Min, Max	100.0, 100.0	33.3, 33.3	100.0, 100.0	50.0, 50.0	100.0, 100.0	33.3, 50.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Global Health Status/QoL

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		14		50	
Mean (SD)	58.10 (20.362)		52.38 (20.524)		56.50 (20.363)	
Median	58.33		50.00		54.17	
Min, Max	16.7, 100.0		16.7, 91.7		16.7, 100.0	
Week 2						
n	36	36	14	14		
Mean (SD)	63.89 (19.107)	5.79 (18.238)	56.55 (17.656)	4.17 (21.620)		
Median	66.67	8.33	58.33	-4.17		
Min, Max	16.7, 100.0	-50.0, 41.7	25.0, 83.3	-25.0, 50.0		

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Global Health Status/QoL

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	36	36	14	14		
Mean (SD)	69.68 (19.534)	11.57 (17.737)	53.57 (22.578)	1.19 (15.627)		
Median	66.67	8.33	50.00	0.00		
Min, Max	25.0, 100.0	-16.7, 50.0	25.0, 100.0	-16.7, 41.7		
Week 8						
n	31	31	13	13		
Mean (SD)	67.74 (21.272)	10.48 (22.357)	58.33 (22.567)	5.13 (21.120)		
Median	75.00	16.67	66.67	0.00		
Min, Max	16.7, 100.0	-41.7, 50.0	16.7, 83.3	-33.3, 41.7		

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Global Health Status/QoL

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	35	35	14	14		
Mean (SD)	70.71 (18.232)	12.38 (15.962)	65.48 (16.296)	13.10 (16.894)		
Median	75.00	8.33	66.67	16.67		
Min, Max	33.3, 100.0	-16.7, 41.7	33.3, 91.7	-16.7, 41.7		
Week 24						
n	35	35	13	13		
Mean (SD)	69.76 (17.860)	11.43 (17.745)	65.38 (20.651)	12.18 (15.447)		
Median	66.67	16.67	66.67	16.67		
Min, Max	33.3, 100.0	-33.3, 41.7	16.7, 100.0	-16.7, 33.3		

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Global Health Status/QoL

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	11	11	40	40
Mean (SD)	72.13 (18.131)	11.21 (14.134)	63.64 (24.231)	9.85 (20.006)	69.79 (20.033)	10.83 (15.698)
Median	66.67	8.33	66.67	16.67	66.67	16.67
Min, Max	33.3, 100.0	-16.7, 33.3	16.7, 100.0	-33.3, 33.3	16.7, 100.0	-33.3, 33.3
Week 56						
n	22	22	7	7	29	29
Mean (SD)	66.67 (19.072)	4.92 (14.240)	63.10 (27.156)	5.95 (23.430)	65.80 (20.815)	5.17 (16.429)
Median	66.67	0.00	66.67	0.00	66.67	0.00
Min, Max	33.3, 100.0	-16.7, 41.7	8.3, 83.3	-25.0, 41.7	8.3, 100.0	-25.0, 41.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Global Health Status/QoL

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	4	4	17	17
Mean (SD)	68.59 (14.495)	5.77 (24.857)	54.17 (28.464)	6.25 (38.112)	65.20 (18.689)	5.88 (27.126)
Median	66.67	0.00	58.33	4.17	66.67	0.00
Min, Max	41.7, 100.0	-50.0, 41.7	16.7, 83.3	-33.3, 50.0	16.7, 100.0	-50.0, 50.0
Week 88						
n	9	9	3	3	12	12
Mean (SD)	74.07 (17.403)	6.48 (13.679)	66.67 (16.667)	19.44 (20.972)	72.22 (16.792)	9.72 (15.825)
Median	83.33	8.33	66.67	16.67	75.00	12.50
Min, Max	41.7, 100.0	-16.7, 25.0	50.0, 83.3	0.0, 41.7	41.7, 100.0	-16.7, 41.7

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Global Health Status/QoL

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	75.00 (22.048)	0.00 (0.000)	83.33 (NA)	41.67 (NA)	77.08 (18.478)	10.42 (20.833)
Median	66.67	0.00	83.33	41.67	75.00	0.00
Min, Max	58.3, 100.0	0.0, 0.0	83.3, 83.3	41.7, 41.7	58.3, 100.0	0.0, 41.7

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Physical Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		14		50	
Mean (SD)	75.19 (19.816)		63.81 (22.144)		72.00 (20.909)	
Median	80.00		66.67		73.33	
Min, Max	33.3, 100.0		26.7, 100.0		26.7, 100.0	
Week 2						
n	35	35	14	14		
Mean (SD)	81.90 (15.724)	7.24 (13.272)	64.76 (20.825)	0.95 (13.551)		
Median	86.67	0.00	66.67	0.00		
Min, Max	40.0, 100.0	-20.0, 46.7	26.7, 100.0	-13.3, 40.0		

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Physical Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	36	36	14	14		
Mean (SD)	87.41 (13.216)	12.22 (16.213)	70.00 (22.149)	6.19 (18.576)		
Median	86.67	6.67	73.33	3.33		
Min, Max	60.0, 100.0	-6.7, 60.0	26.7, 100.0	-26.7, 53.3		
Week 8						
n	31	31	13	13		
Mean (SD)	84.73 (15.318)	10.54 (16.580)	73.85 (17.525)	9.23 (15.525)		
Median	86.67	13.33	73.33	0.00		
Min, Max	33.3, 100.0	-26.7, 46.7	33.3, 100.0	-6.7, 46.7		

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Physical Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	35	35	14	14		
Mean (SD)	88.76 (11.176)	13.14 (14.950)	69.05 (21.341)	5.24 (15.944)		
Median	86.67	6.67	73.33	3.33		
Min, Max	66.7, 100.0	-6.7, 46.7	33.3, 100.0	-20.0, 46.7		
Week 24						
n	35	35	13	13		
Mean (SD)	88.00 (12.711)	12.38 (18.866)	75.90 (23.498)	9.23 (15.040)		
Median	86.67	13.33	80.00	6.67		
Min, Max	53.3, 100.0	-33.3, 60.0	26.7, 100.0	-6.7, 40.0		

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Physical Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	11	11	40	40
Mean (SD)	88.51 (12.071)	10.57 (15.381)	73.33 (25.473)	3.03 (13.119)	84.33 (17.834)	8.50 (15.020)
Median	93.33	6.67	73.33	0.00	86.67	6.67
Min, Max	60.0, 100.0	-13.3, 53.3	33.3, 100.0	-20.0, 26.7	33.3, 100.0	-20.0, 53.3
Week 56						
n	22	22	7	7	29	29
Mean (SD)	86.67 (14.693)	8.79 (17.138)	85.71 (16.069)	8.57 (14.764)	86.44 (14.745)	8.74 (16.340)
Median	90.00	3.33	86.67	13.33	86.67	6.67
Min, Max	53.3, 100.0	-20.0, 60.0	53.3, 100.0	-13.3, 33.3	53.3, 100.0	-20.0, 60.0

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Physical Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	14	14	4	4	18	18
Mean (SD)	82.86 (15.407)	7.14 (19.120)	76.67 (15.870)	11.67 (12.620)	81.48 (15.263)	8.15 (17.647)
Median	86.67	6.67	76.67	16.67	86.67	10.00
Min, Max	53.3, 100.0	-13.3, 60.0	60.0, 93.3	-6.7, 20.0	53.3, 100.0	-13.3, 60.0
Week 88						
n	9	9	3	3	12	12
Mean (SD)	86.67 (14.907)	8.15 (11.915)	84.44 (16.777)	20.00 (17.638)	86.11 (14.622)	11.11 (13.731)
Median	93.33	6.67	86.67	26.67	90.00	6.67
Min, Max	60.0, 100.0	-6.7, 33.3	66.7, 100.0	0.0, 33.3	60.0, 100.0	-6.7, 33.3

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Physical Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	91.11 (15.396)	6.67 (6.667)	86.67 (NA)	33.33 (NA)	90.00 (12.766)	13.33 (14.402)
Median	100.00	6.67	86.67	33.33	93.33	10.00
Min, Max	73.3, 100.0	0.0, 13.3	86.7, 86.7	33.3, 33.3	73.3, 100.0	0.0, 33.3

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Role Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		14		50	
Mean (SD)	64.35 (30.120)		69.05 (29.131)		65.67 (29.625)	
Median	66.67		66.67		66.67	
Min, Max	0.0, 100.0		16.7, 100.0		0.0, 100.0	
Week 2						
n	34	34	14	14		
Mean (SD)	76.96 (26.910)	13.73 (23.018)	67.86 (30.287)	-1.19 (19.019)		
Median	83.33	0.00	66.67	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 33.3		

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Role Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	36	36	14	14		
Mean (SD)	85.19 (20.998)	20.83 (29.107)	70.24 (26.294)	1.19 (19.019)		
Median	100.00	16.67	75.00	0.00		
Min, Max	0.0, 100.0	-66.7, 83.3	16.7, 100.0	-33.3, 50.0		
Week 8						
n	31	31	13	13		
Mean (SD)	82.80 (20.854)	20.43 (23.456)	69.23 (26.217)	-3.85 (31.294)		
Median	83.33	16.67	66.67	0.00		
Min, Max	33.3, 100.0	-16.7, 83.3	16.7, 100.0	-66.7, 66.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Role Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	35	35	14	14		
Mean (SD)	84.76 (18.245)	19.52 (24.417)	71.43 (28.815)	2.38 (29.131)		
Median	100.00	16.67	75.00	0.00		
Min, Max	33.3, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 66.7		
Week 24						
n	35	35	13	13		
Mean (SD)	80.95 (24.965)	15.71 (29.689)	79.49 (23.721)	7.69 (27.735)		
Median	83.33	16.67	83.33	16.67		
Min, Max	0.0, 100.0	-66.7, 83.3	33.3, 100.0	-33.3, 50.0		

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Role Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	11	11	40	40
Mean (SD)	85.06 (17.449)	15.52 (24.369)	74.24 (26.208)	4.55 (13.104)	82.08 (20.460)	12.50 (22.249)
Median	100.00	0.00	66.67	0.00	91.67	0.00
Min, Max	50.0, 100.0	-16.7, 66.7	16.7, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 66.7
Week 56						
n	22	22	7	7	29	29
Mean (SD)	78.03 (19.509)	8.33 (26.602)	85.71 (17.817)	9.52 (26.972)	79.89 (19.096)	8.62 (26.209)
Median	66.67	0.00	100.00	0.00	66.67	0.00
Min, Max	33.3, 100.0	-33.3, 66.7	66.7, 100.0	-33.3, 50.0	33.3, 100.0	-33.3, 66.7

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Role Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	14	14	4	4	18	18
Mean (SD)	80.95 (29.131)	19.05 (24.335)	70.83 (25.000)	8.33 (28.868)	78.70 (27.892)	16.67 (24.918)
Median	100.00	16.67	66.67	8.33	91.67	16.67
Min, Max	0.0, 100.0	-16.7, 66.7	50.0, 100.0	-16.7, 33.3	0.0, 100.0	-16.7, 66.7
Week 88						
n	9	9	3	3	12	12
Mean (SD)	87.04 (20.031)	18.52 (21.155)	83.33 (16.667)	22.22 (38.490)	86.11 (18.577)	19.44 (24.447)
Median	100.00	16.67	83.33	0.00	100.00	8.33
Min, Max	50.0, 100.0	0.0, 50.0	66.7, 100.0	0.0, 66.7	50.0, 100.0	0.0, 66.7

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Role Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	83.33 (28.868)	5.56 (9.623)	66.67 (NA)	50.00 (NA)	79.17 (25.000)	16.67 (23.570)
Median	100.00	0.00	66.67	50.00	83.33	8.33
Min, Max	50.0, 100.0	0.0, 16.7	66.7, 66.7	50.0, 50.0	50.0, 100.0	0.0, 50.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Emotional Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		14		50	
Mean (SD)	78.94 (21.866)		70.83 (29.190)		76.67 (24.105)	
Median	83.33		79.17		83.33	
Min, Max	0.0, 100.0		8.3, 100.0		0.0, 100.0	
Week 2						
n	35	35	14	14		
Mean (SD)	83.81 (17.497)	5.00 (17.410)	73.81 (20.111)	2.98 (12.913)		
Median	91.67	0.00	79.17	0.00		
Min, Max	41.7, 100.0	-25.0, 58.3	33.3, 100.0	-16.7, 25.0		

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Emotional Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	36	36	14	14		
Mean (SD)	85.65 (22.679)	6.71 (18.774)	75.00 (22.408)	4.17 (11.671)		
Median	91.67	0.00	83.33	0.00		
Min, Max	0.0, 100.0	-66.7, 58.3	25.0, 100.0	-8.3, 33.3		
Week 8						
n	31	31	13	13		
Mean (SD)	83.60 (17.806)	7.26 (19.807)	78.21 (22.704)	2.56 (9.851)		
Median	91.67	8.33	83.33	0.00		
Min, Max	41.7, 100.0	-25.0, 58.3	16.7, 100.0	-8.3, 16.7		

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Emotional Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	35	35	14	14		
Mean (SD)	88.33 (15.811)	9.05 (14.627)	73.21 (27.382)	2.38 (12.416)		
Median	100.00	8.33	75.00	0.00		
Min, Max	41.7, 100.0	-8.3, 66.7	0.0, 100.0	-16.7, 25.0		
Week 24						
n	35	35	13	13		
Mean (SD)	85.48 (21.231)	6.19 (20.447)	74.36 (21.099)	3.21 (21.392)		
Median	91.67	0.00	75.00	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	33.3, 100.0	-33.3, 50.0		

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Emotional Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	11	11	40	40
Mean (SD)	86.78 (22.218)	4.89 (20.716)	75.76 (22.191)	6.82 (23.810)	83.75 (22.484)	5.42 (21.313)
Median	100.00	8.33	75.00	0.00	91.67	4.17
Min, Max	0.0, 100.0	-66.7, 58.3	25.0, 100.0	-25.0, 66.7	0.0, 100.0	-66.7, 66.7
Week 56						
n	22	22	7	7	29	29
Mean (SD)	84.47 (23.612)	3.41 (22.072)	75.00 (19.245)	14.29 (31.445)	82.18 (22.683)	6.03 (24.488)
Median	95.83	0.00	83.33	0.00	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 50.0	41.7, 100.0	-16.7, 75.0	0.0, 100.0	-66.7, 75.0

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Emotional Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	4	4	17	17
Mean (SD)	90.38 (13.108)	5.13 (13.411)	54.17 (25.000)	12.50 (17.347)	81.86 (22.290)	6.86 (14.202)
Median	91.67	0.00	58.33	12.50	91.67	0.00
Min, Max	58.3, 100.0	-16.7, 33.3	25.0, 75.0	-8.3, 33.3	25.0, 100.0	-16.7, 33.3
Week 88						
n	9	9	3	3	12	12
Mean (SD)	93.52 (10.848)	6.48 (13.029)	72.22 (33.679)	44.44 (33.679)	88.19 (19.611)	15.97 (24.989)
Median	100.00	0.00	91.67	25.00	91.67	12.50
Min, Max	66.7, 100.0	-8.3, 25.0	33.3, 91.7	25.0, 83.3	33.3, 100.0	-8.3, 83.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Emotional Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	94.44 (9.623)	2.78 (4.811)	91.67 (NA)	83.33 (NA)	93.75 (7.979)	22.92 (40.469)
Median	100.00	0.00	91.67	83.33	95.83	4.17
Min, Max	83.3, 100.0	0.0, 8.3	91.7, 91.7	83.3, 83.3	83.3, 100.0	0.0, 83.3

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Cognitive Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		14		50	
Mean (SD)	77.31 (22.940)		69.05 (30.562)		75.00 (25.254)	
Median	83.33		66.67		83.33	
Min, Max	16.7, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	35	35	14	14		
Mean (SD)	81.43 (17.514)	4.29 (18.675)	77.38 (22.272)	8.33 (23.342)		
Median	83.33	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-16.7, 66.7	16.7, 100.0	-33.3, 66.7		

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Cognitive Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	36	36	14	14		
Mean (SD)	84.72 (17.537)	7.41 (20.097)	76.19 (27.514)	7.14 (24.209)		
Median	83.33	0.00	83.33	0.00		
Min, Max	16.7, 100.0	-50.0, 50.0	0.0, 100.0	-33.3, 50.0		
Week 8						
n	31	31	13	13		
Mean (SD)	81.72 (16.863)	6.99 (19.614)	75.64 (28.557)	6.41 (23.113)		
Median	83.33	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-33.3, 66.7	0.0, 100.0	-33.3, 50.0		

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Cognitive Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	35	35	14	14		
Mean (SD)	85.71 (14.654)	8.10 (16.356)	75.00 (25.944)	5.95 (24.985)		
Median	83.33	0.00	75.00	0.00		
Min, Max	50.0, 100.0	-16.7, 50.0	16.7, 100.0	-33.3, 50.0		
Week 24						
n	35	35	13	13		
Mean (SD)	84.76 (20.361)	7.14 (22.246)	79.49 (21.681)	8.97 (18.777)		
Median	83.33	0.00	83.33	0.00		
Min, Max	0.0, 100.0	-66.7, 50.0	33.3, 100.0	-33.3, 33.3		

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Cognitive Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	11	11	40	40
Mean (SD)	84.48 (20.860)	6.90 (22.056)	74.24 (23.995)	0.00 (16.667)	81.67 (21.943)	5.00 (20.741)
Median	83.33	0.00	66.67	0.00	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 50.0	16.7, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 50.0
Week 56						
n	22	22	7	7	29	29
Mean (SD)	87.88 (19.370)	5.30 (20.821)	69.05 (35.262)	2.38 (17.817)	83.33 (24.801)	4.60 (19.869)
Median	100.00	0.00	83.33	0.00	83.33	0.00
Min, Max	16.7, 100.0	-50.0, 50.0	0.0, 100.0	-16.7, 33.3	0.0, 100.0	-50.0, 50.0

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Cognitive Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	4	4	17	17
Mean (SD)	91.03 (12.938)	7.69 (16.124)	62.50 (31.549)	4.17 (15.957)	84.31 (21.627)	6.86 (15.656)
Median	100.00	0.00	75.00	8.33	83.33	0.00
Min, Max	66.7, 100.0	0.0, 50.0	16.7, 83.3	-16.7, 16.7	16.7, 100.0	-16.7, 50.0
Week 88						
n	9	9	3	3	12	12
Mean (SD)	94.44 (8.333)	7.41 (12.108)	61.11 (41.944)	16.67 (16.667)	86.11 (24.447)	9.72 (13.216)
Median	100.00	0.00	66.67	16.67	100.00	0.00
Min, Max	83.3, 100.0	0.0, 33.3	16.7, 100.0	0.0, 33.3	16.7, 100.0	0.0, 33.3

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Cognitive Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	88.89 (19.245)	0.00 (0.000)	83.33 (NA)	16.67 (NA)	87.50 (15.957)	4.17 (8.333)
Median	100.00	0.00	83.33	16.67	91.67	0.00
Min, Max	66.7, 100.0	0.0, 0.0	83.3, 83.3	16.7, 16.7	66.7, 100.0	0.0, 16.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Social Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	36		14		50	
Mean (SD)	74.07 (27.442)		70.24 (32.803)		73.00 (28.747)	
Median	83.33		83.33		83.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	36	36	14	14		
Mean (SD)	85.65 (19.981)	11.57 (23.845)	70.24 (30.084)	0.00 (14.618)		
Median	100.00	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 83.3	0.0, 100.0	-16.7, 33.3		

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Social Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	36	36	14	14		
Mean (SD)	85.65 (20.375)	11.57 (22.474)	78.57 (24.832)	8.33 (21.434)		
Median	100.00	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-33.3, 83.3	16.7, 100.0	-16.7, 50.0		
Week 8						
n	31	31	13	13		
Mean (SD)	87.63 (20.170)	13.98 (20.681)	75.64 (23.189)	0.00 (15.215)		
Median	100.00	16.67	66.67	0.00		
Min, Max	33.3, 100.0	-16.7, 83.3	16.7, 100.0	-16.7, 33.3		

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Social Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	35	35	14	14		
Mean (SD)	89.05 (17.123)	14.29 (20.269)	73.81 (29.753)	3.57 (14.876)		
Median	100.00	0.00	83.33	0.00		
Min, Max	33.3, 100.0	0.0, 83.3	0.0, 100.0	-16.7, 33.3		
Week 24						
n	35	35	13	13		
Mean (SD)	84.76 (22.277)	10.00 (27.771)	75.64 (23.189)	7.69 (24.167)		
Median	100.00	0.00	66.67	0.00		
Min, Max	0.0, 100.0	-66.7, 83.3	16.7, 100.0	-33.3, 50.0		

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Table GERMANY.2.5.11.7

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab) Modified Randomized Set

Social Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	29	29	11	11	40	40
Mean (SD)	87.36 (17.624)	8.05 (24.243)	71.21 (23.677)	3.03 (29.644)	82.92 (20.495)	6.67 (25.543)
Median	100.00	0.00	83.33	0.00	91.67	0.00
Min, Max	50.0, 100.0	-33.3, 83.3	33.3, 100.0	-66.7, 50.0	33.3, 100.0	-66.7, 83.3
Week 56						
n	22	22	7	7	29	29
Mean (SD)	87.88 (18.674)	9.85 (27.535)	80.95 (14.996)	9.52 (26.972)	86.21 (17.856)	9.77 (26.917)
Median	100.00	0.00	83.33	0.00	100.00	0.00
Min, Max	33.3, 100.0	-33.3, 83.3	66.7, 100.0	-16.7, 66.7	33.3, 100.0	-33.3, 83.3

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Social Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	13	13	3	3	16	16
Mean (SD)	91.03 (16.124)	16.67 (27.217)	94.44 (9.623)	11.11 (9.623)	91.67 (14.907)	15.63 (24.697)
Median	100.00	0.00	100.00	16.67	100.00	8.33
Min, Max	50.0, 100.0	-16.7, 83.3	83.3, 100.0	0.0, 16.7	50.0, 100.0	-16.7, 83.3
Week 88						
n	9	9	3	3	12	12
Mean (SD)	90.74 (14.699)	12.96 (28.599)	88.89 (19.245)	33.33 (28.868)	90.28 (15.006)	18.06 (28.831)
Median	100.00	0.00	100.00	16.67	100.00	16.67
Min, Max	66.7, 100.0	-16.7, 83.3	66.7, 100.0	16.7, 66.7	66.7, 100.0	-16.7, 83.3

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Social Functioning

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	3	3	1	1	4	4
Mean (SD)	88.89 (19.245)	-11.11 (19.245)	66.67 (NA)	66.67 (NA)	83.33 (19.245)	8.33 (41.944)
Median	100.00	0.00	66.67	66.67	83.33	0.00
Min, Max	66.7, 100.0	-33.3, 0.0	66.7, 66.7	66.7, 66.7	66.7, 100.0	-33.3, 66.7

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Table GERMANY.2.5.11.8

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Global Health Status/QoL

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	58.33 (20.412)		56.25 (18.478)		57.64 (18.956)	
Median	50.00		50.00		50.00	
Min, Max	33.3, 91.7		41.7, 83.3		33.3, 91.7	
Week 2						
n	8	8	4	4		
Mean (SD)	64.58 (18.230)	6.25 (18.767)	64.58 (17.180)	8.33 (24.533)		
Median	54.17	4.17	66.67	8.33		
Min, Max	50.0, 91.7	-25.0, 33.3	41.7, 83.3	-16.7, 33.3		

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Global Health Status/QoL

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	8	8	4	4		
Mean (SD)	70.83 (19.920)	12.50 (19.416)	58.33 (9.623)	2.08 (14.232)		
Median	70.83	8.33	58.33	4.17		
Min, Max	50.0, 100.0	-16.7, 41.7	50.0, 66.7	-16.7, 16.7		
Week 8						
n	8	8	4	4		
Mean (SD)	64.58 (21.246)	6.25 (20.774)	64.58 (10.486)	8.33 (21.517)		
Median	54.17	8.33	66.67	8.33		
Min, Max	41.7, 100.0	-33.3, 33.3	50.0, 75.0	-16.7, 33.3		

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Global Health Status/QoL

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	8	8	4	4		
Mean (SD)	69.79 (23.962)	11.46 (23.119)	68.75 (10.486)	12.50 (24.056)		
Median	70.83	8.33	66.67	12.50		
Min, Max	33.3, 100.0	-16.7, 41.7	58.3, 83.3	-16.7, 41.7		
Week 24						
n	8	8	4	4		
Mean (SD)	63.54 (29.526)	5.21 (23.544)	60.42 (18.478)	4.17 (19.837)		
Median	58.33	8.33	66.67	4.17		
Min, Max	16.7, 100.0	-33.3, 33.3	33.3, 75.0	-16.7, 25.0		

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Global Health Status/QoL

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	68.06 (26.043)	11.11 (20.861)	69.44 (4.811)	22.22 (9.623)	68.52 (20.741)	14.81 (18.056)
Median	75.00	12.50	66.67	16.67	66.67	16.67
Min, Max	33.3, 100.0	-16.7, 33.3	66.7, 75.0	16.7, 33.3	33.3, 100.0	-16.7, 33.3
Week 56						
n	4	4	1	1	5	5
Mean (SD)	72.92 (26.680)	8.33 (11.785)	50.00 (NA)	0.00 (NA)	68.33 (25.276)	6.67 (10.865)
Median	70.83	4.17	50.00	0.00	50.00	0.00
Min, Max	50.0, 100.0	0.0, 25.0	50.0, 50.0	0.0, 0.0	50.0, 100.0	0.0, 25.0

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Global Health Status/QoL

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	1	1	0	0	1	1
Mean (SD)	41.67 (NA)	-8.33 (NA)			41.67 (NA)	-8.33 (NA)
Median	41.67	-8.33			41.67	-8.33
Min, Max	41.7, 41.7	-8.3, -8.3			41.7, 41.7	-8.3, -8.3

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Physical Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	74.17 (19.334)		73.33 (14.402)		73.89 (17.164)	
Median	76.67		70.00		70.00	
Min, Max	40.0, 100.0		60.0, 93.3		40.0, 100.0	
Week 2						
n	8	8	4	4		
Mean (SD)	77.50 (12.817)	3.33 (14.693)	73.33 (13.333)	0.00 (5.443)		
Median	73.33	0.00	66.67	0.00		
Min, Max	66.7, 100.0	-20.0, 26.7	66.7, 93.3	-6.7, 6.7		

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Physical Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	8	8	4	4		
Mean (SD)	85.00 (12.724)	10.83 (18.148)	75.00 (13.744)	1.67 (8.389)		
Median	86.67	6.67	73.33	0.00		
Min, Max	60.0, 100.0	-6.7, 46.7	60.0, 93.3	-6.7, 13.3		
Week 8						
n	8	8	4	4		
Mean (SD)	80.00 (14.693)	5.83 (17.252)	76.67 (15.870)	3.33 (3.849)		
Median	83.33	0.00	70.00	3.33		
Min, Max	60.0, 100.0	-6.7, 46.7	66.7, 100.0	0.0, 6.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Physical Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	8	8	4	4		
Mean (SD)	85.00 (13.214)	10.83 (16.690)	71.67 (16.667)	-1.67 (8.389)		
Median	86.67	6.67	70.00	0.00		
Min, Max	66.7, 100.0	-6.7, 46.7	53.3, 93.3	-13.3, 6.7		
Week 24						
n	8	8	4	4		
Mean (SD)	85.83 (15.711)	11.67 (19.760)	73.33 (21.082)	0.00 (7.698)		
Median	90.00	3.33	70.00	0.00		
Min, Max	53.3, 100.0	-6.7, 53.3	53.3, 100.0	-6.7, 6.7		

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Physical Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	84.44 (17.213)	11.11 (18.217)	75.56 (31.505)	0.00 (17.638)	81.48 (21.286)	7.41 (17.778)
Median	90.00	3.33	86.67	6.67	86.67	6.67
Min, Max	60.0, 100.0	0.0, 46.7	40.0, 100.0	-20.0, 13.3	40.0, 100.0	-20.0, 46.7
Week 56						
n	4	4	1	1	5	5
Mean (SD)	76.67 (26.943)	-1.67 (11.386)	93.33 (NA)	0.00 (NA)	80.00 (24.495)	-1.33 (9.888)
Median	76.67	-3.33	93.33	0.00	93.33	0.00
Min, Max	53.3, 100.0	-13.3, 13.3	93.3, 93.3	0.0, 0.0	53.3, 100.0	-13.3, 13.3

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Physical Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	1	1	0	0	1	1
Mean (SD)	60.00 (NA)	-6.67 (NA)			60.00 (NA)	-6.67 (NA)
Median	60.00	-6.67			60.00	-6.67
Min, Max	60.0, 60.0	-6.7, -6.7			60.0, 60.0	-6.7, -6.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Role Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	66.67 (23.570)		87.50 (15.957)		73.61 (22.983)	
Median	66.67		91.67		66.67	
Min, Max	16.7, 100.0		66.7, 100.0		16.7, 100.0	
Week 2						
n	8	8	4	4		
Mean (SD)	70.83 (31.810)	4.17 (14.773)	83.33 (19.245)	-4.17 (20.972)		
Median	75.00	0.00	83.33	0.00		
Min, Max	0.0, 100.0	-16.7, 33.3	66.7, 100.0	-33.3, 16.7		

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Role Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	8	8	4	4		
Mean (SD)	85.42 (5.893)	18.75 (24.296)	83.33 (13.608)	-4.17 (8.333)		
Median	83.33	16.67	83.33	0.00		
Min, Max	83.3, 100.0	-16.7, 66.7	66.7, 100.0	-16.7, 0.0		
Week 8						
n	8	8	4	4		
Mean (SD)	83.33 (12.599)	16.67 (17.817)	70.83 (20.972)	-16.67 (13.608)		
Median	83.33	16.67	66.67	-16.67		
Min, Max	66.7, 100.0	0.0, 50.0	50.0, 100.0	-33.3, 0.0		

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Role Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	8	8	4	4		
Mean (SD)	91.67 (8.909)	25.00 (19.920)	70.83 (8.333)	-16.67 (13.608)		
Median	91.67	16.67	66.67	-16.67		
Min, Max	83.3, 100.0	0.0, 66.7	66.7, 83.3	-33.3, 0.0		
Week 24						
n	8	8	4	4		
Mean (SD)	85.42 (24.296)	18.75 (25.877)	75.00 (21.517)	-12.50 (25.000)		
Median	100.00	25.00	75.00	-16.67		
Min, Max	33.3, 100.0	-33.3, 50.0	50.0, 100.0	-33.3, 16.7		

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Role Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	86.11 (16.387)	19.44 (26.701)	77.78 (19.245)	-5.56 (9.623)	83.33 (16.667)	11.11 (25.000)
Median	91.67	8.33	66.67	0.00	83.33	0.00
Min, Max	66.7, 100.0	0.0, 66.7	66.7, 100.0	-16.7, 0.0	66.7, 100.0	-16.7, 66.7
Week 56						
n	4	4	1	1	5	5
Mean (SD)	83.33 (19.245)	8.33 (16.667)	100.00 (NA)	0.00 (NA)	86.67 (18.257)	6.67 (14.907)
Median	83.33	0.00	100.00	0.00	100.00	0.00
Min, Max	66.7, 100.0	0.0, 33.3	100.0, 100.0	0.0, 0.0	66.7, 100.0	0.0, 33.3

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Role Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	1	1	0	0	1	1
Mean (SD)	100.00 (NA)	33.33 (NA)			100.00 (NA)	33.33 (NA)
Median	100.00	33.33			100.00	33.33
Min, Max	100.0, 100.0	33.3, 33.3			100.0, 100.0	33.3, 33.3

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Emotional Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	83.33 (11.785)		89.58 (10.486)		85.42 (11.307)	
Median	83.33		91.67		83.33	
Min, Max	66.7, 100.0		75.0, 100.0		66.7, 100.0	
Week 2						
n	8	8	4	4		
Mean (SD)	83.33 (16.667)	0.00 (12.599)	83.33 (11.785)	-6.25 (7.979)		
Median	83.33	-4.17	79.17	-4.17		
Min, Max	58.3, 100.0	-16.7, 16.7	75.0, 100.0	-16.7, 0.0		

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Emotional Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	8	8	4	4		
Mean (SD)	86.46 (12.550)	3.13 (7.634)	83.33 (13.608)	-6.25 (4.167)		
Median	83.33	0.00	83.33	-8.33		
Min, Max	66.7, 100.0	-8.3, 16.7	66.7, 100.0	-8.3, 0.0		
Week 8						
n	8	8	4	4		
Mean (SD)	83.33 (17.817)	0.00 (12.599)	81.25 (10.486)	-8.33 (0.000)		
Median	83.33	0.00	83.33	-8.33		
Min, Max	50.0, 100.0	-25.0, 16.7	66.7, 91.7	-8.3, -8.3		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Emotional Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	8	8	4	4		
Mean (SD)	87.50 (16.667)	4.17 (10.911)	79.17 (14.434)	-10.42 (7.979)		
Median	95.83	4.17	75.00	-12.50		
Min, Max	58.3, 100.0	-16.7, 16.7	66.7, 100.0	-16.7, 0.0		
Week 24						
n	8	8	4	4		
Mean (SD)	87.50 (16.667)	4.17 (10.911)	81.25 (14.232)	-8.33 (6.804)		
Median	95.83	4.17	79.17	-8.33		
Min, Max	58.3, 100.0	-16.7, 16.7	66.7, 100.0	-16.7, 0.0		

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Emotional Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	93.06 (9.742)	8.33 (10.541)	75.00 (14.434)	-13.89 (9.623)	87.04 (13.889)	0.93 (14.699)
Median	95.83	4.17	66.67	-8.33	91.67	0.00
Min, Max	75.0, 100.0	0.0, 25.0	66.7, 91.7	-25.0, -8.3	66.7, 100.0	-25.0, 25.0
Week 56						
n	4	4	1	1	5	5
Mean (SD)	85.42 (23.936)	0.00 (18.002)	75.00 (NA)	0.00 (NA)	83.33 (21.246)	0.00 (15.590)
Median	95.83	4.17	75.00	0.00	91.67	0.00
Min, Max	50.0, 100.0	-25.0, 16.7	75.0, 75.0	0.0, 0.0	50.0, 100.0	-25.0, 16.7

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Emotional Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	1	1	0	0	1	1
Mean (SD)	91.67 (NA)	8.33 (NA)			91.67 (NA)	8.33 (NA)
Median	91.67	8.33			91.67	8.33
Min, Max	91.7, 91.7	8.3, 8.3			91.7, 91.7	8.3, 8.3

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Cognitive Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	75.00 (23.570)		83.33 (13.608)		77.78 (20.515)	
Median	83.33		83.33		83.33	
Min, Max	33.3, 100.0		66.7, 100.0		33.3, 100.0	
Week 2						
n	8	8	4	4		
Mean (SD)	72.92 (17.678)	-2.08 (10.681)	79.17 (15.957)	-4.17 (20.972)		
Median	75.00	0.00	75.00	0.00		
Min, Max	50.0, 100.0	-16.7, 16.7	66.7, 100.0	-33.3, 16.7		

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Cognitive Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	8	8	4	4		
Mean (SD)	85.42 (13.909)	10.42 (21.708)	79.17 (15.957)	-4.17 (20.972)		
Median	83.33	0.00	75.00	0.00		
Min, Max	66.7, 100.0	-16.7, 50.0	66.7, 100.0	-33.3, 16.7		
Week 8						
n	8	8	4	4		
Mean (SD)	83.33 (15.430)	8.33 (17.817)	79.17 (15.957)	-4.17 (20.972)		
Median	83.33	0.00	75.00	0.00		
Min, Max	66.7, 100.0	-16.7, 33.3	66.7, 100.0	-33.3, 16.7		

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Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	8	8	4	4		
Mean (SD)	79.17 (23.146)	4.17 (17.252)	79.17 (15.957)	-4.17 (25.000)		
Median	83.33	0.00	75.00	0.00		
Min, Max	33.3, 100.0	-16.7, 33.3	66.7, 100.0	-33.3, 16.7		
Week 24						
n	8	8	4	4		
Mean (SD)	81.25 (24.296)	6.25 (19.796)	83.33 (13.608)	0.00 (23.570)		
Median	91.67	0.00	83.33	8.33		
Min, Max	33.3, 100.0	-16.7, 33.3	66.7, 100.0	-33.3, 16.7		

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Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	88.89 (13.608)	16.67 (18.257)	72.22 (9.623)	-11.11 (19.245)	83.33 (14.434)	7.41 (22.222)
Median	91.67	16.67	66.67	0.00	83.33	0.00
Min, Max	66.7, 100.0	0.0, 50.0	66.7, 83.3	-33.3, 0.0	66.7, 100.0	-33.3, 50.0
Week 56						
n	4	4	1	1	5	5
Mean (SD)	87.50 (25.000)	4.17 (8.333)	83.33 (NA)	16.67 (NA)	86.67 (21.731)	6.67 (9.129)
Median	100.00	0.00	83.33	16.67	100.00	0.00
Min, Max	50.0, 100.0	0.0, 16.7	83.3, 83.3	16.7, 16.7	50.0, 100.0	0.0, 16.7

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Cognitive Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	1	1	0	0	1	1
Mean (SD)	100.00 (NA)	0.00 (NA)			100.00 (NA)	0.00 (NA)
Median	100.00	0.00			100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0			100.0, 100.0	0.0, 0.0

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Social Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	75.00 (25.198)		95.83 (8.333)		81.94 (22.983)	
Median	75.00		100.00		91.67	
Min, Max	33.3, 100.0		83.3, 100.0		33.3, 100.0	
Week 2						
n	8	8	4	4		
Mean (SD)	85.42 (24.296)	10.42 (19.796)	91.67 (16.667)	-4.17 (8.333)		
Median	100.00	0.00	100.00	0.00		
Min, Max	33.3, 100.0	0.0, 50.0	66.7, 100.0	-16.7, 0.0		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Social Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	8	8	4	4		
Mean (SD)	89.58 (15.269)	14.58 (18.767)	95.83 (8.333)	0.00 (13.608)		
Median	100.00	8.33	100.00	0.00		
Min, Max	66.7, 100.0	0.0, 50.0	83.3, 100.0	-16.7, 16.7		
Week 8						
n	8	8	4	4		
Mean (SD)	85.42 (18.767)	10.42 (17.678)	91.67 (9.623)	-4.17 (8.333)		
Median	91.67	0.00	91.67	0.00		
Min, Max	50.0, 100.0	0.0, 50.0	83.3, 100.0	-16.7, 0.0		

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Social Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	8	8	4	4		
Mean (SD)	89.58 (15.269)	14.58 (20.774)	91.67 (16.667)	-4.17 (8.333)		
Median	100.00	0.00	100.00	0.00		
Min, Max	66.7, 100.0	0.0, 50.0	66.7, 100.0	-16.7, 0.0		
Week 24						
n	8	8	4	4		
Mean (SD)	87.50 (17.252)	12.50 (23.146)	91.67 (16.667)	-4.17 (20.972)		
Median	100.00	0.00	100.00	0.00		
Min, Max	66.7, 100.0	-16.7, 50.0	66.7, 100.0	-33.3, 16.7		

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Social Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	86.11 (22.153)	5.56 (25.092)	72.22 (34.694)	-22.22 (38.490)	81.48 (25.610)	-3.70 (30.932)
Median	100.00	0.00	83.33	0.00	100.00	0.00
Min, Max	50.0, 100.0	-33.3, 33.3	33.3, 100.0	-66.7, 0.0	33.3, 100.0	-66.7, 33.3
Week 56						
n	4	4	1	1	5	5
Mean (SD)	83.33 (33.333)	-4.17 (34.359)	100.00 (NA)	0.00 (NA)	86.67 (29.814)	-3.33 (29.814)
Median	100.00	0.00	100.00	0.00	100.00	0.00
Min, Max	33.3, 100.0	-50.0, 33.3	100.0, 100.0	0.0, 0.0	33.3, 100.0	-50.0, 33.3

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Social Functioning

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	1	1	0	0	1	1
Mean (SD)	100.00 (NA)	0.00 (NA)			100.00 (NA)	0.00 (NA)
Median	100.00	0.00			100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0			100.0, 100.0	0.0, 0.0

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Global Health Status/QoL

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	49		20		69	
Mean (SD)	58.33 (20.341)		55.00 (17.812)		57.37 (19.571)	
Median	66.67		58.33		58.33	
Min, Max	16.7, 100.0		16.7, 91.7		16.7, 100.0	
Week 2						
n	49	49	21	19		
Mean (SD)	63.78 (19.584)	5.44 (17.476)	57.14 (15.654)	2.19 (17.968)		
Median	66.67	8.33	58.33	0.00		
Min, Max	16.7, 100.0	-50.0, 41.7	25.0, 83.3	-25.0, 50.0		

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Global Health Status/QoL

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	49	49	21	20		
Mean (SD)	66.16 (19.574)	7.82 (16.174)	55.56 (23.174)	-0.83 (17.291)		
Median	66.67	0.00	50.00	0.00		
Min, Max	25.0, 100.0	-16.7, 50.0	25.0, 100.0	-41.7, 41.7		
Week 8						
n	37	37	15	14		
Mean (SD)	67.79 (22.323)	9.46 (23.171)	57.22 (24.974)	0.00 (22.169)		
Median	66.67	8.33	66.67	0.00		
Min, Max	16.7, 100.0	-58.3, 50.0	16.7, 83.3	-33.3, 41.7		

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Global Health Status/QoL

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	44	44	21	20		
Mean (SD)	69.89 (18.081)	12.31 (14.093)	61.11 (17.938)	5.42 (17.579)		
Median	66.67	16.67	66.67	0.00		
Min, Max	33.3, 100.0	-16.7, 33.3	33.3, 91.7	-25.0, 33.3		
Week 24						
n	41	41	20	19		
Mean (SD)	69.92 (17.066)	10.98 (15.969)	67.50 (19.289)	11.84 (14.250)		
Median	66.67	16.67	66.67	8.33		
Min, Max	33.3, 100.0	-33.3, 41.7	16.7, 100.0	-8.3, 41.7		

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Global Health Status/QoL

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Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	40	40	18	18	58	58
Mean (SD)	68.75 (18.848)	10.00 (15.585)	61.57 (24.941)	3.70 (17.901)	66.52 (20.971)	8.05 (16.443)
Median	66.67	8.33	66.67	4.17	66.67	8.33
Min, Max	33.3, 100.0	-41.7, 33.3	16.7, 100.0	-33.3, 33.3	16.7, 100.0	-41.7, 33.3
Week 56						
n	30	30	16	15	46	45
Mean (SD)	66.11 (17.770)	7.22 (16.914)	61.98 (21.075)	1.11 (21.563)	64.67 (18.855)	5.19 (18.574)
Median	66.67	0.00	66.67	0.00	66.67	0.00
Min, Max	33.3, 100.0	-16.7, 41.7	8.3, 83.3	-25.0, 41.7	8.3, 100.0	-25.0, 41.7

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Global Health Status/QoL

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	19	19	12	11	31	30
Mean (SD)	71.05 (14.795)	8.77 (22.986)	62.50 (22.891)	2.27 (25.025)	67.74 (18.477)	6.39 (23.538)
Median	66.67	8.33	66.67	0.00	66.67	4.17
Min, Max	33.3, 100.0	-50.0, 41.7	16.7, 91.7	-33.3, 50.0	16.7, 100.0	-50.0, 50.0
Week 88						
n	14	14	8	8	22	22
Mean (SD)	74.40 (15.143)	8.33 (15.676)	61.46 (22.244)	4.17 (23.987)	69.70 (18.642)	6.82 (18.658)
Median	75.00	12.50	66.67	4.17	66.67	8.33
Min, Max	41.7, 100.0	-16.7, 33.3	16.7, 83.3	-33.3, 41.7	16.7, 100.0	-33.3, 41.7

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Global Health Status/QoL

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	4	4	2	2	6	6
Mean (SD)	81.25 (21.916)	8.33 (16.667)	79.17 (5.893)	25.00 (23.570)	80.56 (17.213)	13.89 (18.758)
Median	83.33	0.00	79.17	25.00	79.17	4.17
Min, Max	58.3, 100.0	0.0, 33.3	75.0, 83.3	8.3, 41.7	58.3, 100.0	0.0, 41.7

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Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.11.8

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Physical Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	49		20		69	
Mean (SD)	73.20 (18.929)		63.67 (21.464)		70.43 (20.016)	
Median	80.00		66.67		73.33	
Min, Max	33.3, 100.0		26.7, 100.0		26.7, 100.0	
Week 2						
n	48	48	21	19		
Mean (SD)	79.17 (18.431)	6.39 (11.666)	65.40 (19.162)	1.40 (13.068)		
Median	86.67	0.00	66.67	0.00		
Min, Max	26.7, 100.0	-13.3, 46.7	26.7, 100.0	-13.3, 40.0		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

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Physical Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	49	49	21	20		
Mean (SD)	84.08 (15.859)	10.88 (14.187)	65.40 (22.073)	0.67 (18.844)		
Median	86.67	6.67	66.67	0.00		
Min, Max	40.0, 100.0	-20.0, 60.0	26.7, 100.0	-26.7, 53.3		
Week 8						
n	37	37	15	14		
Mean (SD)	84.32 (15.887)	10.63 (14.269)	68.89 (22.206)	4.76 (19.468)		
Median	86.67	13.33	73.33	0.00		
Min, Max	33.3, 100.0	-26.7, 40.0	20.0, 100.0	-26.7, 46.7		

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Physical Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	44	44	21	20		
Mean (SD)	85.15 (15.741)	11.21 (13.160)	65.08 (21.282)	0.67 (19.090)		
Median	86.67	6.67	73.33	0.00		
Min, Max	26.7, 100.0	-6.7, 40.0	20.0, 100.0	-33.3, 46.7		
Week 24						
n	41	41	20	19		
Mean (SD)	85.85 (14.847)	11.22 (16.428)	78.67 (22.747)	12.63 (13.127)		
Median	86.67	13.33	86.67	13.33		
Min, Max	40.0, 100.0	-33.3, 60.0	26.7, 100.0	-6.7, 40.0		

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Physical Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	40	40	18	18	58	58
Mean (SD)	83.67 (17.796)	9.00 (14.911)	73.33 (24.628)	5.56 (14.326)	80.46 (20.514)	7.93 (14.694)
Median	86.67	10.00	73.33	3.33	86.67	6.67
Min, Max	13.3, 100.0	-26.7, 53.3	20.0, 100.0	-26.7, 26.7	13.3, 100.0	-26.7, 53.3
Week 56						
n	30	30	16	15	46	45
Mean (SD)	84.22 (14.802)	10.67 (14.814)	78.33 (17.470)	6.67 (15.119)	82.17 (15.842)	9.33 (14.866)
Median	86.67	13.33	80.00	13.33	86.67	13.33
Min, Max	46.7, 100.0	-20.0, 60.0	46.7, 100.0	-20.0, 33.3	46.7, 100.0	-20.0, 60.0

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Physical Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	20	20	12	11	32	31
Mean (SD)	85.67 (13.027)	11.00 (16.653)	75.00 (25.126)	6.06 (18.001)	81.67 (18.856)	9.25 (17.012)
Median	86.67	13.33	80.00	13.33	86.67	13.33
Min, Max	53.3, 100.0	-13.3, 60.0	6.7, 100.0	-40.0, 20.0	6.7, 100.0	-40.0, 60.0
Week 88						
n	14	14	8	8	22	22
Mean (SD)	88.10 (12.858)	10.95 (10.972)	74.17 (24.543)	7.50 (17.252)	83.03 (18.713)	9.70 (13.290)
Median	90.00	6.67	76.67	3.33	86.67	6.67
Min, Max	60.0, 100.0	-6.7, 33.3	33.3, 100.0	-13.3, 33.3	33.3, 100.0	-13.3, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Physical Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	4	4	2	2	6	6
Mean (SD)	91.67 (12.620)	8.33 (6.383)	90.00 (4.714)	20.00 (18.856)	91.11 (10.037)	12.22 (11.483)
Median	96.67	10.00	90.00	20.00	93.33	10.00
Min, Max	73.3, 100.0	0.0, 13.3	86.7, 93.3	6.7, 33.3	73.3, 100.0	0.0, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Role Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	49		20		69	
Mean (SD)	67.35 (28.251)		58.33 (27.836)		64.73 (28.229)	
Median	66.67		66.67		66.67	
Min, Max	0.0, 100.0		16.7, 100.0		0.0, 100.0	
Week 2						
n	47	47	20	18		
Mean (SD)	75.89 (27.977)	9.22 (24.772)	65.00 (28.562)	3.70 (16.721)		
Median	83.33	0.00	66.67	0.00		
Min, Max	0.0, 100.0	-50.0, 66.7	0.0, 100.0	-33.3, 33.3		

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Role Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	49	49	20	19		
Mean (SD)	80.61 (25.763)	13.27 (28.050)	65.00 (27.519)	3.51 (28.098)		
Median	100.00	0.00	66.67	0.00		
Min, Max	0.0, 100.0	-66.7, 83.3	16.7, 100.0	-33.3, 83.3		
Week 8						
n	37	37	14	13		
Mean (SD)	81.08 (22.278)	14.41 (24.896)	66.67 (28.495)	1.28 (31.521)		
Median	83.33	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 83.3	16.7, 100.0	-66.7, 66.7		

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Role Functioning

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Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	44	44	20	19		
Mean (SD)	80.30 (23.645)	12.50 (24.675)	68.33 (31.484)	8.77 (35.732)		
Median	91.67	0.00	83.33	0.00		
Min, Max	0.0, 100.0	-33.3, 66.7	0.0, 100.0	-66.7, 66.7		
Week 24						
n	41	41	20	19		
Mean (SD)	76.02 (29.356)	7.72 (30.299)	74.17 (31.750)	14.91 (38.044)		
Median	83.33	0.00	83.33	33.33		
Min, Max	0.0, 100.0	-66.7, 83.3	0.0, 100.0	-100.0, 66.7		

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Role Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	40	40	18	18	58	58
Mean (SD)	79.17 (25.806)	8.75 (26.145)	66.67 (36.604)	7.41 (27.548)	75.29 (29.821)	8.33 (26.352)
Median	91.67	0.00	75.00	8.33	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-50.0, 50.0	0.0, 100.0	-66.7, 66.7
Week 56						
n	30	30	16	15	46	45
Mean (SD)	73.33 (26.839)	3.33 (28.834)	80.21 (15.176)	17.78 (27.794)	75.72 (23.493)	8.15 (29.003)
Median	66.67	0.00	75.00	16.67	66.67	0.00
Min, Max	0.0, 100.0	-83.3, 66.7	66.7, 100.0	-33.3, 66.7	0.0, 100.0	-83.3, 66.7

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Source: ADaM.ADSL, ADaM.ADQS

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Role Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	20	20	11	10	31	30
Mean (SD)	80.00 (27.889)	14.17 (26.087)	77.27 (21.438)	20.00 (25.820)	79.03 (25.447)	16.11 (25.702)
Median	100.00	8.33	83.33	25.00	83.33	16.67
Min, Max	0.0, 100.0	-33.3, 66.7	50.0, 100.0	-16.7, 66.7	0.0, 100.0	-33.3, 66.7
Week 88						
n	14	14	8	8	22	22
Mean (SD)	85.71 (22.510)	13.10 (20.856)	70.83 (27.817)	12.50 (29.209)	80.30 (25.006)	12.88 (23.532)
Median	100.00	0.00	75.00	0.00	91.67	0.00
Min, Max	33.3, 100.0	-16.7, 50.0	16.7, 100.0	-16.7, 66.7	16.7, 100.0	-16.7, 66.7

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Role Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	4	4	2	2	6	6
Mean (SD)	87.50 (25.000)	4.17 (8.333)	83.33 (23.570)	33.33 (23.570)	86.11 (22.153)	13.89 (19.484)
Median	100.00	0.00	83.33	33.33	100.00	8.33
Min, Max	50.0, 100.0	0.0, 16.7	66.7, 100.0	16.7, 50.0	50.0, 100.0	0.0, 50.0

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Emotional Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	49		20		69	
Mean (SD)	79.08 (23.700)		70.83 (25.291)		76.69 (24.278)	
Median	83.33		79.17		83.33	
Min, Max	0.0, 100.0		8.3, 100.0		0.0, 100.0	
Week 2						
n	48	48	21	19		
Mean (SD)	84.55 (18.593)	5.56 (15.973)	72.22 (22.257)	2.63 (15.970)		
Median	91.67	0.00	75.00	0.00		
Min, Max	33.3, 100.0	-25.0, 58.3	33.3, 100.0	-33.3, 25.0		

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Emotional Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	49	49	21	20		
Mean (SD)	83.67 (23.690)	4.59 (20.203)	76.19 (23.465)	4.17 (17.832)		
Median	91.67	0.00	83.33	0.00		
Min, Max	0.0, 100.0	-66.7, 58.3	25.0, 100.0	-33.3, 33.3		
Week 8						
n	37	37	15	14		
Mean (SD)	85.59 (16.970)	8.11 (18.161)	83.89 (22.815)	9.52 (12.167)		
Median	91.67	8.33	91.67	12.50		
Min, Max	41.7, 100.0	-25.0, 58.3	16.7, 100.0	-16.7, 33.3		

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Emotional Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	44	44	21	20		
Mean (SD)	87.31 (19.061)	8.14 (14.655)	73.41 (25.361)	2.50 (18.945)		
Median	100.00	4.17	75.00	4.17		
Min, Max	16.7, 100.0	-8.3, 66.7	0.0, 100.0	-50.0, 33.3		
Week 24						
n	41	41	20	19		
Mean (SD)	84.35 (23.437)	5.69 (19.934)	80.83 (18.945)	10.09 (18.753)		
Median	91.67	0.00	83.33	8.33		
Min, Max	0.0, 100.0	-66.7, 66.7	33.3, 100.0	-33.3, 50.0		

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Emotional Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	40	40	18	18	58	58
Mean (SD)	84.17 (22.865)	4.17 (19.971)	78.24 (19.625)	7.41 (21.559)	82.33 (21.914)	5.17 (20.342)
Median	91.67	4.17	75.00	4.17	91.67	4.17
Min, Max	0.0, 100.0	-66.7, 58.3	25.0, 100.0	-25.0, 66.7	0.0, 100.0	-66.7, 66.7
Week 56						
n	30	30	16	15	46	45
Mean (SD)	81.94 (24.279)	4.17 (22.611)	79.17 (18.758)	9.44 (26.701)	80.98 (22.337)	5.93 (23.878)
Median	91.67	0.00	83.33	0.00	87.50	0.00
Min, Max	0.0, 100.0	-66.7, 50.0	41.7, 100.0	-16.7, 75.0	0.0, 100.0	-66.7, 75.0

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Emotional Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	19	19	12	11	31	30
Mean (SD)	90.79 (13.578)	5.26 (16.483)	76.39 (24.317)	9.09 (17.262)	85.22 (19.450)	6.67 (16.580)
Median	100.00	0.00	75.00	8.33	91.67	4.17
Min, Max	58.3, 100.0	-33.3, 33.3	25.0, 100.0	-25.0, 33.3	25.0, 100.0	-33.3, 33.3
Week 88						
n	14	14	8	8	22	22
Mean (SD)	91.07 (12.431)	6.55 (10.928)	77.08 (24.701)	16.67 (35.355)	85.98 (18.614)	10.23 (22.703)
Median	95.83	4.17	87.50	20.83	91.67	8.33
Min, Max	66.7, 100.0	-8.3, 25.0	33.3, 100.0	-33.3, 83.3	33.3, 100.0	-33.3, 83.3

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Emotional Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	4	4	2	2	6	6
Mean (SD)	95.83 (8.333)	2.08 (4.167)	62.50 (41.248)	25.00 (82.496)	84.72 (26.043)	9.72 (38.879)
Median	100.00	0.00	62.50	25.00	95.83	0.00
Min, Max	83.3, 100.0	0.0, 8.3	33.3, 91.7	-33.3, 83.3	33.3, 100.0	-33.3, 83.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Cognitive Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	49		20		69	
Mean (SD)	79.25 (22.702)		67.50 (27.823)		75.85 (24.677)	
Median	83.33		66.67		83.33	
Min, Max	16.7, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	48	48	20	19		
Mean (SD)	84.72 (17.810)	5.56 (17.643)	72.50 (29.753)	6.14 (24.976)		
Median	83.33	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-16.7, 66.7	0.0, 100.0	-33.3, 66.7		

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Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.11.8

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Cognitive Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	49	49	21	20		
Mean (SD)	84.35 (18.449)	5.10 (18.068)	69.84 (31.894)	1.67 (25.876)		
Median	83.33	0.00	83.33	0.00		
Min, Max	16.7, 100.0	-50.0, 50.0	0.0, 100.0	-33.3, 50.0		
Week 8						
n	37	37	15	14		
Mean (SD)	84.23 (16.642)	7.66 (18.255)	76.67 (29.412)	13.10 (19.805)		
Median	83.33	0.00	83.33	8.33		
Min, Max	33.3, 100.0	-33.3, 66.7	0.0, 100.0	-16.7, 50.0		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Cognitive Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	44	44	21	20		
Mean (SD)	85.98 (16.449)	7.58 (15.017)	74.60 (27.698)	6.67 (22.556)		
Median	83.33	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-16.7, 50.0	16.7, 100.0	-33.3, 50.0		
Week 24						
n	41	41	20	19		
Mean (SD)	82.52 (24.424)	4.47 (21.090)	77.50 (23.740)	8.77 (17.004)		
Median	83.33	0.00	83.33	0.00		
Min, Max	0.0, 100.0	-66.7, 50.0	33.3, 100.0	-16.7, 33.3		

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Cognitive Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	40	40	18	18	58	58
Mean (SD)	81.25 (23.323)	3.33 (20.395)	70.37 (29.459)	-0.93 (19.363)	77.87 (25.628)	2.01 (20.009)
Median	83.33	0.00	75.00	0.00	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 50.0	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 50.0
Week 56						
n	30	30	16	15	46	45
Mean (SD)	83.89 (22.095)	4.44 (20.498)	72.92 (32.131)	0.00 (21.822)	80.07 (26.204)	2.96 (20.807)
Median	91.67	0.00	83.33	0.00	83.33	0.00
Min, Max	16.7, 100.0	-50.0, 50.0	0.0, 100.0	-50.0, 33.3	0.0, 100.0	-50.0, 50.0

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Cognitive Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	19	19	11	10	30	29
Mean (SD)	91.23 (12.872)	7.02 (16.021)	74.24 (31.059)	6.67 (16.102)	85.00 (22.468)	6.90 (15.759)
Median	100.00	0.00	83.33	8.33	100.00	0.00
Min, Max	66.7, 100.0	-16.7, 50.0	16.7, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 50.0
Week 88						
n	14	14	8	8	22	22
Mean (SD)	94.05 (10.555)	7.14 (12.599)	60.42 (38.768)	0.00 (26.726)	81.82 (29.054)	4.55 (18.674)
Median	100.00	0.00	75.00	0.00	100.00	0.00
Min, Max	66.7, 100.0	0.0, 33.3	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Cognitive Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	4	4	2	2	6	6
Mean (SD)	91.67 (16.667)	0.00 (0.000)	58.33 (35.355)	-8.33 (35.355)	80.56 (26.701)	-2.78 (16.387)
Median	100.00	0.00	58.33	-8.33	91.67	0.00
Min, Max	66.7, 100.0	0.0, 0.0	33.3, 83.3	-33.3, 16.7	33.3, 100.0	-33.3, 16.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Social Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	49		20		69	
Mean (SD)	75.51 (26.154)		64.17 (31.657)		72.22 (28.103)	
Median	83.33		66.67		83.33	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	49	49	20	18		
Mean (SD)	81.97 (20.648)	6.46 (22.775)	67.50 (28.855)	3.70 (21.047)		
Median	83.33	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-50.0, 83.3	0.0, 100.0	-33.3, 50.0		

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Social Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	49	49	20	19		
Mean (SD)	82.31 (21.626)	6.80 (21.495)	67.50 (27.293)	3.51 (24.582)		
Median	100.00	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 83.3	0.0, 100.0	-50.0, 50.0		
Week 8						
n	37	37	14	13		
Mean (SD)	86.49 (19.578)	9.91 (23.720)	77.38 (24.114)	8.97 (18.777)		
Median	100.00	0.00	66.67	16.67		
Min, Max	33.3, 100.0	-50.0, 83.3	16.7, 100.0	-16.7, 33.3		

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Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	44	44	21	20		
Mean (SD)	85.98 (19.669)	9.85 (20.748)	63.49 (32.326)	-2.50 (28.753)		
Median	100.00	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 83.3	0.0, 100.0	-83.3, 33.3		
Week 24						
n	41	41	19	18		
Mean (SD)	80.89 (26.761)	4.47 (26.621)	71.93 (26.089)	10.19 (24.347)		
Median	100.00	0.00	66.67	16.67		
Min, Max	0.0, 100.0	-66.7, 83.3	0.0, 100.0	-33.3, 50.0		

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	40	40	18	18	58	58
Mean (SD)	79.17 (25.248)	2.08 (27.783)	59.26 (36.703)	-4.63 (46.725)	72.99 (30.401)	0.00 (34.483)
Median	83.33	0.00	66.67	8.33	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 83.3	0.0, 100.0	-100.0, 50.0	0.0, 100.0	-100.0, 83.3
Week 56						
n	30	30	16	15	46	45
Mean (SD)	83.89 (20.287)	9.44 (23.846)	76.04 (19.215)	8.89 (29.457)	81.16 (20.064)	9.26 (25.514)
Median	100.00	0.00	75.00	0.00	83.33	0.00
Min, Max	33.3, 100.0	-33.3, 83.3	33.3, 100.0	-50.0, 66.7	33.3, 100.0	-50.0, 83.3

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Social Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	19	19	11	10	30	29
Mean (SD)	87.72 (19.909)	17.54 (25.744)	80.30 (25.624)	1.67 (32.820)	85.00 (22.037)	12.07 (28.832)
Median	100.00	16.67	83.33	16.67	100.00	16.67
Min, Max	33.3, 100.0	-16.7, 83.3	16.7, 100.0	-83.3, 33.3	16.7, 100.0	-83.3, 83.3
Week 88						
n	14	14	8	8	22	22
Mean (SD)	86.90 (16.249)	11.90 (23.956)	79.17 (17.252)	10.42 (33.259)	84.09 (16.649)	11.36 (26.917)
Median	100.00	0.00	66.67	16.67	91.67	8.33
Min, Max	66.7, 100.0	-16.7, 83.3	66.7, 100.0	-33.3, 66.7	66.7, 100.0	-33.3, 83.3

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.11.8

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Social Functioning

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	4	4	2	2	6	6
Mean (SD)	91.67 (16.667)	0.00 (27.217)	83.33 (23.570)	58.33 (11.785)	88.89 (17.213)	19.44 (37.143)
Median	100.00	0.00	83.33	58.33	100.00	16.67
Min, Max	66.7, 100.0	-33.3, 33.3	66.7, 100.0	50.0, 66.7	66.7, 100.0	-33.3, 66.7

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Table GERMANY.2.5.11.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Global Health Status/QoL

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	60.98 (19.482)		51.67 (22.153)		58.07 (20.466)	
Median	66.67		50.00		54.17	
Min, Max	33.3, 100.0		16.7, 91.7		16.7, 100.0	
Week 2						
n	22	22	10	10		
Mean (SD)	66.29 (17.151)	5.30 (14.444)	65.83 (14.933)	14.17 (20.806)		
Median	66.67	4.17	66.67	16.67		
Min, Max	33.3, 91.7	-25.0, 33.3	41.7, 83.3	-16.7, 50.0		

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Table GERMANY.2.5.11.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Global Health Status/QoL

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	10		
Mean (SD)	68.56 (19.233)	7.58 (16.846)	59.17 (24.985)	7.50 (17.765)		
Median	66.67	0.00	58.33	8.33		
Min, Max	25.0, 100.0	-16.7, 41.7	25.0, 100.0	-16.7, 41.7		
Week 8						
n	18	18	10	10		
Mean (SD)	71.76 (17.183)	9.72 (18.798)	62.50 (22.652)	10.83 (20.431)		
Median	70.83	8.33	66.67	16.67		
Min, Max	41.7, 100.0	-33.3, 41.7	16.7, 83.3	-16.7, 41.7		

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Table GERMANY.2.5.11.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Global Health Status/QoL

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	10		
Mean (SD)	73.33 (19.978)	11.25 (17.990)	70.00 (14.803)	18.33 (17.480)		
Median	75.00	8.33	66.67	20.83		
Min, Max	33.3, 100.0	-16.7, 41.7	50.0, 91.7	-16.7, 41.7		
Week 24						
n	20	20	9	9		
Mean (SD)	70.00 (21.357)	7.92 (18.824)	65.74 (27.146)	12.96 (19.145)		
Median	70.83	8.33	66.67	16.67		
Min, Max	16.7, 100.0	-33.3, 33.3	16.7, 100.0	-16.7, 41.7		

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Table GERMANY.2.5.11.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1
and Period 2 and Long Term Extension by Race
Modified Randomized Set

Global Health Status/QoL

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	7	7	27	27
Mean (SD)	72.08 (19.734)	11.25 (16.284)	65.48 (26.540)	11.90 (21.440)	70.37 (21.350)	11.42 (17.319)
Median	70.83	12.50	66.67	16.67	66.67	16.67
Min, Max	33.3, 100.0	-16.7, 33.3	16.7, 100.0	-33.3, 33.3	16.7, 100.0	-33.3, 33.3
Week 56						
n	16	16	5	5	21	21
Mean (SD)	68.75 (16.527)	8.33 (13.944)	61.67 (33.124)	5.00 (24.008)	67.06 (20.829)	7.54 (16.224)
Median	66.67	8.33	83.33	0.00	66.67	8.33
Min, Max	50.0, 100.0	-16.7, 41.7	8.3, 83.3	-25.0, 33.3	8.3, 100.0	-25.0, 41.7

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Table GERMANY.2.5.11.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Global Health Status/QoL

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	3	3	12	12
Mean (SD)	72.22 (16.137)	13.89 (20.833)	61.11 (38.490)	13.89 (42.763)	69.44 (22.000)	13.89 (25.459)
Median	66.67	16.67	83.33	25.00	70.83	20.83
Min, Max	41.7, 100.0	-16.7, 41.7	16.7, 83.3	-33.3, 50.0	16.7, 100.0	-33.3, 50.0
Week 88						
n	7	7	2	2	9	9
Mean (SD)	70.24 (15.105)	11.90 (16.567)	66.67 (23.570)	20.83 (5.893)	69.44 (15.590)	13.89 (15.023)
Median	66.67	16.67	66.67	20.83	66.67	16.67
Min, Max	41.7, 83.3	-16.7, 33.3	50.0, 83.3	16.7, 25.0	41.7, 83.3	-16.7, 33.3

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Table GERMANY.2.5.11.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Global Health Status/QoL

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	0	0	2	2
Mean (SD)	83.33 (23.570)	16.67 (23.570)			83.33 (23.570)	16.67 (23.570)
Median	83.33	16.67			83.33	16.67
Min, Max	66.7, 100.0	0.0, 33.3			66.7, 100.0	0.0, 33.3

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Table GERMANY.2.5.11.9

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Physical Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	78.79 (17.413)		66.67 (24.343)		75.00 (20.249)	
Median	80.00		70.00		76.67	
Min, Max	40.0, 100.0		26.7, 100.0		26.7, 100.0	
Week 2						
n	22	22	10	10		
Mean (SD)	83.64 (14.508)	4.85 (12.203)	73.33 (19.116)	6.67 (12.958)		
Median	86.67	0.00	70.00	3.33		
Min, Max	53.3, 100.0	-20.0, 33.3	33.3, 100.0	-6.7, 40.0		

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Table GERMANY.2.5.11.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Physical Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	10		
Mean (SD)	86.36 (12.927)	7.58 (13.847)	78.00 (14.072)	11.33 (17.512)		
Median	86.67	0.00	80.00	6.67		
Min, Max	53.3, 100.0	-6.7, 46.7	53.3, 100.0	-6.7, 53.3		
Week 8						
n	18	18	10	10		
Mean (SD)	84.07 (13.163)	5.93 (13.312)	79.33 (14.555)	12.67 (16.163)		
Median	86.67	0.00	73.33	6.67		
Min, Max	60.0, 100.0	-6.7, 46.7	60.0, 100.0	0.0, 46.7		

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Table GERMANY.2.5.11.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1
and Period 2 and Long Term Extension by Race
Modified Randomized Set

Physical Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	10		
Mean (SD)	89.67 (11.337)	9.33 (15.202)	75.33 (18.338)	8.67 (16.040)		
Median	93.33	3.33	76.67	6.67		
Min, Max	66.7, 100.0	-6.7, 46.7	40.0, 100.0	-13.3, 46.7		
Week 24						
n	20	20	9	9		
Mean (SD)	88.67 (12.255)	8.33 (15.121)	78.52 (26.200)	7.41 (12.222)		
Median	90.00	3.33	93.33	6.67		
Min, Max	53.3, 100.0	-6.7, 53.3	26.7, 100.0	-6.7, 26.7		

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Physical Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	7	7	27	27
Mean (SD)	87.33 (13.139)	8.33 (13.134)	83.81 (24.300)	5.71 (16.069)	86.42 (16.276)	7.65 (13.676)
Median	90.00	3.33	100.00	6.67	93.33	6.67
Min, Max	60.0, 100.0	-6.7, 46.7	40.0, 100.0	-20.0, 26.7	40.0, 100.0	-20.0, 46.7
Week 56						
n	16	16	5	5	21	21
Mean (SD)	83.75 (15.000)	5.42 (13.601)	92.00 (8.692)	9.33 (8.944)	85.71 (14.029)	6.35 (12.557)
Median	86.67	3.33	93.33	13.33	86.67	6.67
Min, Max	53.3, 100.0	-20.0, 26.7	80.0, 100.0	0.0, 20.0	53.3, 100.0	-20.0, 26.7

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Modified Randomized Set

Physical Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	3	3	12	12
Mean (SD)	82.96 (13.789)	8.15 (12.373)	93.33 (6.667)	20.00 (0.000)	85.56 (12.975)	11.11 (11.835)
Median	80.00	6.67	93.33	20.00	86.67	13.33
Min, Max	60.0, 100.0	-13.3, 26.7	86.7, 100.0	20.0, 20.0	60.0, 100.0	-13.3, 26.7
Week 88						
n	7	7	2	2	9	9
Mean (SD)	83.81 (12.683)	8.57 (9.974)	100.00 (0.000)	23.33 (4.714)	87.41 (13.100)	11.85 (10.943)
Median	86.67	6.67	100.00	23.33	86.67	6.67
Min, Max	60.0, 100.0	-6.7, 26.7	100.0, 100.0	20.0, 26.7	60.0, 100.0	-6.7, 26.7

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Modified Randomized Set

Physical Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	0	0	2	2
Mean (SD)	96.67 (4.714)	10.00 (4.714)			96.67 (4.714)	10.00 (4.714)
Median	96.67	10.00			96.67	10.00
Min, Max	93.3, 100.0	6.7, 13.3			93.3, 100.0	6.7, 13.3

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Modified Randomized Set

Role Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	74.24 (23.417)		78.33 (22.292)		75.52 (22.791)	
Median	66.67		75.00		66.67	
Min, Max	16.7, 100.0		33.3, 100.0		16.7, 100.0	
Week 2						
n	22	22	10	10		
Mean (SD)	83.33 (24.667)	9.09 (19.739)	81.67 (18.342)	3.33 (21.943)		
Median	100.00	0.00	83.33	8.33		
Min, Max	0.0, 100.0	-16.7, 66.7	50.0, 100.0	-33.3, 33.3		

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Modified Randomized Set

Role Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	10		
Mean (SD)	88.64 (13.979)	14.39 (22.593)	78.33 (19.325)	0.00 (22.222)		
Median	91.67	0.00	83.33	0.00		
Min, Max	50.0, 100.0	-16.7, 66.7	33.3, 100.0	-33.3, 50.0		
Week 8						
n	18	18	10	10		
Mean (SD)	85.19 (13.873)	13.89 (17.386)	76.67 (23.831)	-1.67 (36.388)		
Median	83.33	8.33	75.00	0.00		
Min, Max	66.7, 100.0	0.0, 50.0	33.3, 100.0	-66.7, 66.7		

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Role Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	10		
Mean (SD)	90.83 (12.653)	15.83 (25.635)	80.00 (21.943)	1.67 (32.820)		
Median	100.00	16.67	83.33	-8.33		
Min, Max	66.7, 100.0	-33.3, 66.7	33.3, 100.0	-33.3, 66.7		
Week 24						
n	20	20	9	9		
Mean (SD)	86.67 (18.417)	11.67 (23.632)	81.48 (25.610)	-1.85 (26.932)		
Median	100.00	0.00	100.00	0.00		
Min, Max	33.3, 100.0	-33.3, 50.0	33.3, 100.0	-33.3, 33.3		

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Role Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	7	7	27	27
Mean (SD)	86.67 (15.858)	11.67 (22.361)	85.71 (17.817)	2.38 (14.996)	86.42 (16.038)	9.26 (20.844)
Median	100.00	0.00	100.00	0.00	100.00	0.00
Min, Max	66.7, 100.0	-16.7, 66.7	66.7, 100.0	-16.7, 33.3	66.7, 100.0	-16.7, 66.7
Week 56						
n	16	16	5	5	21	21
Mean (SD)	82.29 (16.632)	7.29 (18.226)	93.33 (14.907)	6.67 (27.889)	84.92 (16.587)	7.14 (20.119)
Median	75.00	0.00	100.00	0.00	100.00	0.00
Min, Max	66.7, 100.0	-33.3, 50.0	66.7, 100.0	-33.3, 33.3	66.7, 100.0	-33.3, 50.0

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Modified Randomized Set

Role Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	3	3	12	12
Mean (SD)	92.59 (14.699)	16.67 (25.000)	88.89 (9.623)	11.11 (25.459)	91.67 (13.295)	15.28 (24.056)
Median	100.00	0.00	83.33	16.67	100.00	8.33
Min, Max	66.7, 100.0	-16.7, 50.0	83.3, 100.0	-16.7, 33.3	66.7, 100.0	-16.7, 50.0
Week 88						
n	7	7	2	2	9	9
Mean (SD)	78.57 (24.934)	0.00 (9.623)	100.00 (0.000)	16.67 (23.570)	83.33 (23.570)	3.70 (13.889)
Median	83.33	0.00	100.00	16.67	100.00	0.00
Min, Max	33.3, 100.0	-16.7, 16.7	100.0, 100.0	0.0, 33.3	33.3, 100.0	-16.7, 33.3

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Modified Randomized Set

Role Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	0	0	2	2
Mean (SD)	100.00 (0.000)	0.00 (0.000)			100.00 (0.000)	0.00 (0.000)
Median	100.00	0.00			100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0			100.0, 100.0	0.0, 0.0

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Emotional Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	84.09 (17.234)		75.83 (26.774)		81.51 (20.602)	
Median	87.50		79.17		83.33	
Min, Max	33.3, 100.0		8.3, 100.0		8.3, 100.0	
Week 2						
n	22	22	10	10		
Mean (SD)	87.50 (14.717)	3.41 (15.140)	79.17 (18.942)	3.33 (14.272)		
Median	91.67	0.00	83.33	0.00		
Min, Max	58.3, 100.0	-16.7, 58.3	33.3, 100.0	-16.7, 25.0		

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Modified Randomized Set

Emotional Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	10		
Mean (SD)	90.15 (11.962)	6.06 (15.036)	79.17 (22.652)	3.33 (13.147)		
Median	91.67	0.00	83.33	0.00		
Min, Max	66.7, 100.0	-8.3, 58.3	25.0, 100.0	-8.3, 33.3		
Week 8						
n	18	18	10	10		
Mean (SD)	87.04 (16.475)	6.48 (17.516)	78.33 (24.595)	2.50 (13.637)		
Median	95.83	4.17	83.33	0.00		
Min, Max	50.0, 100.0	-25.0, 58.3	16.7, 100.0	-8.3, 33.3		

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and Period 2 and Long Term Extension by Race
Modified Randomized Set

Emotional Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	10		
Mean (SD)	94.17 (12.121)	8.33 (16.885)	78.33 (20.108)	2.50 (16.221)		
Median	100.00	4.17	75.00	0.00		
Min, Max	58.3, 100.0	-16.7, 66.7	33.3, 100.0	-16.7, 33.3		
Week 24						
n	20	20	9	9		
Mean (SD)	91.67 (12.681)	5.83 (13.813)	74.07 (23.365)	-2.78 (20.833)		
Median	100.00	0.00	83.33	-8.33		
Min, Max	58.3, 100.0	-16.7, 41.7	33.3, 100.0	-33.3, 33.3		

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Modified Randomized Set

Emotional Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	7	7	27	27
Mean (SD)	94.58 (9.473)	10.00 (15.198)	75.00 (26.352)	0.00 (19.245)	89.51 (17.387)	7.41 (16.559)
Median	100.00	4.17	75.00	-8.33	100.00	0.00
Min, Max	66.7, 100.0	0.0, 58.3	25.0, 100.0	-25.0, 33.3	25.0, 100.0	-25.0, 58.3
Week 56						
n	16	16	5	5	21	21
Mean (SD)	89.06 (15.129)	5.21 (18.226)	76.67 (21.570)	10.00 (22.361)	86.11 (17.146)	6.35 (18.802)
Median	95.83	0.00	83.33	0.00	91.67	0.00
Min, Max	50.0, 100.0	-25.0, 50.0	41.7, 100.0	-16.7, 33.3	41.7, 100.0	-25.0, 50.0

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Modified Randomized Set

Emotional Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	3	3	12	12
Mean (SD)	94.44 (8.333)	7.41 (13.466)	66.67 (38.188)	13.89 (20.972)	87.50 (21.760)	9.03 (14.847)
Median	100.00	0.00	75.00	16.67	95.83	4.17
Min, Max	75.0, 100.0	-8.3, 33.3	25.0, 100.0	-8.3, 33.3	25.0, 100.0	-8.3, 33.3
Week 88						
n	7	7	2	2	9	9
Mean (SD)	89.29 (13.363)	3.57 (10.603)	66.67 (47.140)	29.17 (5.893)	84.26 (22.609)	9.26 (14.699)
Median	91.67	0.00	66.67	29.17	91.67	0.00
Min, Max	66.7, 100.0	-8.3, 25.0	33.3, 100.0	25.0, 33.3	33.3, 100.0	-8.3, 33.3

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Modified Randomized Set

Emotional Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	0	0	2	2
Mean (SD)	100.00 (0.000)	0.00 (0.000)			100.00 (0.000)	0.00 (0.000)
Median	100.00	0.00			100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0			100.0, 100.0	0.0, 0.0

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Modified Randomized Set

Cognitive Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	83.33 (18.545)		66.67 (35.136)		78.13 (25.554)	
Median	83.33		75.00		83.33	
Min, Max	33.3, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	22	22	10	10		
Mean (SD)	84.09 (16.649)	0.76 (10.883)	78.33 (26.117)	11.67 (26.117)		
Median	83.33	0.00	83.33	8.33		
Min, Max	50.0, 100.0	-16.7, 33.3	16.7, 100.0	-33.3, 66.7		

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Cognitive Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	10		
Mean (SD)	87.88 (13.784)	4.55 (16.412)	78.33 (31.476)	11.67 (26.117)		
Median	91.67	0.00	91.67	0.00		
Min, Max	66.7, 100.0	-16.7, 50.0	0.0, 100.0	-33.3, 50.0		
Week 8						
n	18	18	10	10		
Mean (SD)	85.19 (13.873)	5.56 (16.169)	78.33 (31.476)	11.67 (26.117)		
Median	83.33	0.00	91.67	0.00		
Min, Max	66.7, 100.0	-16.7, 33.3	0.0, 100.0	-33.3, 50.0		

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Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	10		
Mean (SD)	87.50 (17.832)	4.17 (14.178)	80.00 (26.989)	13.33 (26.989)		
Median	100.00	0.00	91.67	16.67		
Min, Max	33.3, 100.0	-16.7, 33.3	16.7, 100.0	-33.3, 50.0		
Week 24						
n	20	20	9	9		
Mean (SD)	86.67 (18.417)	3.33 (15.858)	79.63 (24.689)	11.11 (22.048)		
Median	100.00	0.00	83.33	16.67		
Min, Max	33.3, 100.0	-16.7, 33.3	33.3, 100.0	-33.3, 33.3		

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Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	7	7	27	27
Mean (SD)	90.00 (13.680)	6.67 (16.579)	73.81 (28.637)	0.00 (21.517)	85.80 (19.450)	4.94 (17.792)
Median	100.00	0.00	83.33	0.00	100.00	0.00
Min, Max	66.7, 100.0	-33.3, 50.0	16.7, 100.0	-33.3, 33.3	16.7, 100.0	-33.3, 50.0
Week 56						
n	16	16	5	5	21	21
Mean (SD)	91.67 (13.608)	3.13 (12.500)	73.33 (41.833)	6.67 (19.003)	87.30 (23.514)	3.97 (13.849)
Median	100.00	0.00	83.33	0.00	100.00	0.00
Min, Max	50.0, 100.0	-16.7, 33.3	0.0, 100.0	-16.7, 33.3	0.0, 100.0	-16.7, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Cognitive Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	3	3	12	12
Mean (SD)	96.30 (7.349)	3.70 (7.349)	66.67 (44.096)	11.11 (25.459)	88.89 (23.925)	5.56 (12.975)
Median	100.00	0.00	83.33	16.67	100.00	0.00
Min, Max	83.3, 100.0	0.0, 16.7	16.7, 100.0	-16.7, 33.3	16.7, 100.0	-16.7, 33.3
Week 88						
n	7	7	2	2	9	9
Mean (SD)	92.86 (13.113)	0.00 (0.000)	58.33 (58.926)	25.00 (11.785)	85.19 (28.191)	5.56 (11.785)
Median	100.00	0.00	58.33	25.00	100.00	0.00
Min, Max	66.7, 100.0	0.0, 0.0	16.7, 100.0	16.7, 33.3	16.7, 100.0	0.0, 33.3

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Modified Randomized Set

Cognitive Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	0	0	2	2
Mean (SD)	100.00 (0.000)	0.00 (0.000)			100.00 (0.000)	0.00 (0.000)
Median	100.00	0.00			100.00	0.00
Min, Max	100.0, 100.0	0.0, 0.0			100.0, 100.0	0.0, 0.0

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Social Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	75.76 (25.577)		83.33 (20.787)		78.13 (24.111)	
Median	75.00		83.33		83.33	
Min, Max	16.7, 100.0		33.3, 100.0		16.7, 100.0	
Week 2						
n	22	22	10	10		
Mean (SD)	84.85 (21.150)	9.09 (22.258)	86.67 (15.316)	3.33 (18.922)		
Median	100.00	0.00	91.67	0.00		
Min, Max	33.3, 100.0	-16.7, 83.3	66.7, 100.0	-16.7, 33.3		

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Modified Randomized Set

Social Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	22	22	10	10		
Mean (SD)	87.12 (18.496)	11.36 (22.647)	93.33 (11.653)	10.00 (21.082)		
Median	100.00	0.00	100.00	8.33		
Min, Max	33.3, 100.0	-16.7, 83.3	66.7, 100.0	-16.7, 50.0		
Week 8						
n	18	18	10	10		
Mean (SD)	87.96 (14.913)	9.26 (18.277)	86.67 (15.316)	3.33 (18.922)		
Median	91.67	0.00	91.67	0.00		
Min, Max	50.0, 100.0	-16.7, 50.0	66.7, 100.0	-16.7, 33.3		

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Modified Randomized Set

Social Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	20	20	10	10		
Mean (SD)	90.83 (13.760)	12.50 (22.862)	86.67 (21.943)	3.33 (13.147)		
Median	100.00	0.00	100.00	0.00		
Min, Max	66.7, 100.0	0.0, 83.3	33.3, 100.0	-16.7, 33.3		
Week 24						
n	20	20	9	9		
Mean (SD)	89.17 (15.555)	10.83 (23.740)	87.04 (16.197)	5.56 (26.352)		
Median	100.00	0.00	100.00	0.00		
Min, Max	66.7, 100.0	-16.7, 83.3	66.7, 100.0	-33.3, 50.0		

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Social Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	7	7	27	27
Mean (SD)	85.83 (20.430)	8.33 (27.306)	83.33 (23.570)	-2.38 (31.074)	85.19 (20.844)	5.56 (28.118)
Median	100.00	0.00	83.33	0.00	100.00	0.00
Min, Max	33.3, 100.0	-33.3, 83.3	33.3, 100.0	-66.7, 33.3	33.3, 100.0	-66.7, 83.3
Week 56						
n	16	16	5	5	21	21
Mean (SD)	86.46 (20.384)	10.42 (27.806)	93.33 (9.129)	10.00 (14.907)	88.10 (18.366)	10.32 (24.987)
Median	100.00	0.00	100.00	0.00	100.00	0.00
Min, Max	33.3, 100.0	-50.0, 83.3	83.3, 100.0	0.0, 33.3	33.3, 100.0	-50.0, 83.3

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Social Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	3	3	12	12
Mean (SD)	94.44 (11.785)	25.93 (29.001)	100.00 (0.000)	22.22 (9.623)	95.83 (10.360)	25.00 (25.126)
Median	100.00	33.33	100.00	16.67	100.00	25.00
Min, Max	66.7, 100.0	0.0, 83.3	100.0, 100.0	16.7, 33.3	66.7, 100.0	0.0, 83.3
Week 88						
n	7	7	2	2	9	9
Mean (SD)	76.19 (16.265)	16.67 (31.914)	100.00 (0.000)	25.00 (11.785)	81.48 (17.568)	18.52 (28.191)
Median	66.67	0.00	100.00	25.00	66.67	0.00
Min, Max	66.7, 100.0	0.0, 83.3	100.0, 100.0	16.7, 33.3	66.7, 100.0	0.0, 83.3

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Social Functioning

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	2	2	0	0	2	2
Mean (SD)	100.00 (0.000)	16.67 (23.570)			100.00 (0.000)	16.67 (23.570)
Median	100.00	16.67			100.00	16.67
Min, Max	100.0, 100.0	0.0, 33.3			100.0, 100.0	0.0, 33.3

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and Period 2 and Long Term Extension by Race
Modified Randomized Set

Global Health Status/QoL

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		10		38	
Mean (SD)	58.33 (21.636)		61.67 (11.915)		59.21 (19.451)	
Median	62.50		66.67		66.67	
Min, Max	16.7, 100.0		33.3, 75.0		16.7, 100.0	
Week 2						
n	28	28	10	9		
Mean (SD)	64.29 (20.769)	5.95 (17.252)	51.67 (12.910)	-8.33 (11.785)		
Median	66.67	4.17	54.17	-8.33		
Min, Max	33.3, 100.0	-41.7, 33.3	25.0, 66.7	-25.0, 8.3		

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Global Health Status/QoL

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	28	28	10	10		
Mean (SD)	66.67 (19.772)	8.33 (15.876)	54.17 (19.345)	-7.50 (15.933)		
Median	66.67	4.17	54.17	-8.33		
Min, Max	33.3, 100.0	-16.7, 41.7	25.0, 83.3	-41.7, 16.7		
Week 8						
n	22	22	7	7		
Mean (SD)	65.15 (23.799)	7.58 (23.697)	55.95 (18.456)	-5.95 (17.156)		
Median	66.67	8.33	58.33	0.00		
Min, Max	16.7, 100.0	-58.3, 41.7	33.3, 83.3	-33.3, 16.7		

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Global Health Status/QoL

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	25	25	10	10		
Mean (SD)	67.67 (19.884)	11.33 (14.609)	60.00 (16.102)	-1.67 (15.113)		
Median	66.67	16.67	58.33	0.00		
Min, Max	33.3, 100.0	-16.7, 33.3	33.3, 83.3	-25.0, 16.7		
Week 24						
n	22	22	10	10		
Mean (SD)	68.94 (19.276)	10.23 (17.046)	70.00 (11.249)	8.33 (13.029)		
Median	66.67	12.50	66.67	8.33		
Min, Max	33.3, 100.0	-33.3, 33.3	50.0, 83.3	-8.3, 33.3		

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Table GERMANY.2.5.11.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1
and Period 2 and Long Term Extension by Race
Modified Randomized Set

Global Health Status/QoL

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	10	10	29	29
Mean (SD)	68.42 (21.078)	9.21 (16.873)	65.83 (20.203)	4.17 (14.829)	67.53 (20.454)	7.47 (16.114)
Median	66.67	8.33	66.67	4.17	66.67	8.33
Min, Max	33.3, 100.0	-41.7, 33.3	33.3, 100.0	-16.7, 25.0	33.3, 100.0	-41.7, 33.3
Week 56						
n	13	13	8	8	21	21
Mean (SD)	68.59 (19.589)	5.13 (17.526)	58.33 (12.599)	-6.25 (14.605)	64.68 (17.659)	0.79 (17.059)
Median	66.67	0.00	66.67	0.00	66.67	0.00
Min, Max	33.3, 100.0	-16.7, 33.3	33.3, 66.7	-25.0, 16.7	33.3, 100.0	-25.0, 33.3

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Global Health Status/QoL

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	6	6	14	14
Mean (SD)	67.71 (19.127)	-2.08 (23.884)	62.50 (14.672)	-4.17 (14.672)	65.48 (16.939)	-2.98 (19.776)
Median	66.67	0.00	58.33	-8.33	66.67	0.00
Min, Max	33.3, 91.7	-50.0, 25.0	50.0, 83.3	-16.7, 16.7	33.3, 91.7	-50.0, 25.0
Week 88						
n	6	6	4	4	10	10
Mean (SD)	81.94 (13.351)	5.56 (16.387)	64.58 (10.486)	-4.17 (10.758)	75.00 (14.699)	1.67 (14.593)
Median	83.33	8.33	66.67	-4.17	70.83	0.00
Min, Max	66.7, 100.0	-16.7, 25.0	50.0, 75.0	-16.7, 8.3	50.0, 100.0	-16.7, 25.0

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Modified Randomized Set

Global Health Status/QoL

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	100.00 (NA)	0.00 (NA)	75.00 (NA)	8.33 (NA)	87.50 (17.678)	4.17 (5.893)
Median	100.00	0.00	75.00	8.33	87.50	4.17
Min, Max	100.0, 100.0	0.0, 0.0	75.0, 75.0	8.3, 8.3	75.0, 100.0	0.0, 8.3

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Modified Randomized Set

Physical Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		10		38	
Mean (SD)	71.19 (18.597)		68.67 (14.072)		70.53 (17.373)	
Median	66.67		66.67		66.67	
Min, Max	33.3, 100.0		40.0, 86.7		33.3, 100.0	
Week 2						
n	27	27	10	9		
Mean (SD)	75.31 (20.070)	4.94 (9.354)	64.67 (16.939)	-3.70 (6.759)		
Median	80.00	0.00	66.67	0.00		
Min, Max	26.7, 100.0	-13.3, 20.0	26.7, 86.7	-13.3, 6.7		

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Modified Randomized Set

Physical Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	28	28	10	10		
Mean (SD)	82.38 (17.234)	11.19 (13.705)	64.00 (20.894)	-4.67 (11.780)		
Median	86.67	13.33	63.33	-6.67		
Min, Max	40.0, 100.0	-20.0, 46.7	33.3, 100.0	-26.7, 13.3		
Week 8						
n	22	22	7	7		
Mean (SD)	83.03 (17.302)	10.91 (16.271)	62.86 (17.995)	-2.86 (10.079)		
Median	83.33	13.33	66.67	0.00		
Min, Max	33.3, 100.0	-26.7, 40.0	33.3, 86.7	-20.0, 13.3		

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Physical Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	25	25	10	10		
Mean (SD)	82.67 (18.053)	11.20 (12.281)	66.67 (15.713)	-2.00 (12.590)		
Median	86.67	13.33	70.00	0.00		
Min, Max	26.7, 100.0	-6.7, 33.3	40.0, 93.3	-33.3, 13.3		
Week 24						
n	22	22	10	10		
Mean (SD)	85.15 (15.524)	12.73 (12.667)	84.67 (15.412)	16.00 (14.125)		
Median	86.67	13.33	86.67	13.33		
Min, Max	40.0, 100.0	-6.7, 40.0	53.3, 100.0	-6.7, 40.0		

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Physical Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	10	10	29	29
Mean (SD)	80.00 (21.999)	6.67 (15.713)	73.33 (20.123)	4.67 (10.909)	77.70 (21.253)	5.98 (14.068)
Median	80.00	13.33	73.33	3.33	80.00	6.67
Min, Max	13.3, 100.0	-26.7, 26.7	33.3, 100.0	-6.7, 26.7	13.3, 100.0	-26.7, 26.7
Week 56						
n	13	13	8	8	21	21
Mean (SD)	84.10 (17.751)	10.26 (9.276)	75.00 (19.437)	1.67 (11.127)	80.63 (18.488)	6.98 (10.641)
Median	86.67	13.33	76.67	0.00	80.00	13.33
Min, Max	46.7, 100.0	-6.7, 20.0	46.7, 100.0	-13.3, 13.3	46.7, 100.0	-13.3, 20.0

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Modified Randomized Set

Physical Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	6	6	15	15
Mean (SD)	86.67 (11.055)	8.15 (13.654)	75.56 (12.413)	5.56 (9.813)	82.22 (12.515)	7.11 (11.943)
Median	86.67	13.33	73.33	3.33	86.67	6.67
Min, Max	73.3, 100.0	-13.3, 26.7	60.0, 93.3	-6.7, 20.0	60.0, 100.0	-13.3, 26.7
Week 88						
n	6	6	4	4	10	10
Mean (SD)	96.67 (5.578)	14.44 (12.939)	68.33 (17.533)	-1.67 (6.383)	85.33 (18.271)	8.00 (13.259)
Median	100.00	16.67	63.33	-3.33	93.33	3.33
Min, Max	86.7, 100.0	0.0, 33.3	53.3, 93.3	-6.7, 6.7	53.3, 100.0	-6.7, 33.3

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Physical Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	100.00 (NA)	0.00 (NA)	93.33 (NA)	6.67 (NA)	96.67 (4.714)	3.33 (4.714)
Median	100.00	0.00	93.33	6.67	96.67	3.33
Min, Max	100.0, 100.0	0.0, 0.0	93.3, 93.3	6.7, 6.7	93.3, 100.0	0.0, 6.7

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Table GERMANY.2.5.11.9

Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Role Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		10		38	
Mean (SD)	63.10 (28.093)		55.00 (24.907)		60.96 (27.201)	
Median	66.67		66.67		66.67	
Min, Max	0.0, 100.0		16.7, 83.3		0.0, 100.0	
Week 2						
n	26	26	10	9		
Mean (SD)	69.87 (30.557)	8.33 (24.608)	55.00 (28.382)	-1.85 (10.015)		
Median	75.00	16.67	66.67	0.00		
Min, Max	0.0, 100.0	-50.0, 50.0	0.0, 100.0	-16.7, 16.7		

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Modified Randomized Set

Role Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	28	28	10	10		
Mean (SD)	78.57 (26.394)	15.48 (26.422)	63.33 (26.989)	8.33 (29.659)		
Median	91.67	16.67	66.67	0.00		
Min, Max	0.0, 100.0	-50.0, 66.7	16.7, 100.0	-33.3, 83.3		
Week 8						
n	22	22	7	7		
Mean (SD)	78.79 (23.672)	15.91 (29.312)	50.00 (21.517)	-4.76 (15.853)		
Median	83.33	16.67	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 83.3	16.7, 66.7	-33.3, 16.7		

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Role Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	25	25	10	10		
Mean (SD)	77.33 (25.404)	14.00 (24.851)	71.67 (26.117)	16.67 (31.427)		
Median	66.67	16.67	83.33	16.67		
Min, Max	0.0, 100.0	-33.3, 66.7	16.7, 100.0	-33.3, 66.7		
Week 24						
n	22	22	10	10		
Mean (SD)	75.00 (28.982)	11.36 (29.271)	85.00 (14.593)	30.00 (18.922)		
Median	83.33	16.67	83.33	33.33		
Min, Max	0.0, 100.0	-50.0, 50.0	66.7, 100.0	0.0, 66.7		

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Role Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	10	10	29	29
Mean (SD)	74.56 (33.040)	7.02 (30.589)	73.33 (32.584)	18.33 (19.954)	74.14 (32.301)	10.92 (27.558)
Median	100.00	0.00	83.33	16.67	83.33	16.67
Min, Max	0.0, 100.0	-66.7, 50.0	16.7, 100.0	-16.7, 50.0	0.0, 100.0	-66.7, 50.0
Week 56						
n	13	13	8	8	21	21
Mean (SD)	71.79 (34.950)	0.00 (32.632)	81.25 (13.909)	22.92 (21.708)	75.40 (28.684)	8.73 (30.559)
Median	83.33	0.00	83.33	16.67	83.33	0.00
Min, Max	0.0, 100.0	-83.3, 50.0	66.7, 100.0	0.0, 66.7	0.0, 100.0	-83.3, 66.7

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Modified Randomized Set

Role Functioning

White

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	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	9	9	6	6	15	15
Mean (SD)	72.22 (35.355)	9.26 (25.154)	77.78 (25.092)	22.22 (29.187)	74.44 (30.775)	14.44 (26.627)
Median	83.33	16.67	83.33	25.00	83.33	16.67
Min, Max	0.0, 100.0	-33.3, 50.0	50.0, 100.0	-16.7, 66.7	0.0, 100.0	-33.3, 66.7
Week 88						
n	6	6	4	4	10	10
Mean (SD)	100.00 (0.000)	27.78 (22.771)	66.67 (13.608)	4.17 (20.972)	86.67 (18.922)	18.33 (24.152)
Median	100.00	33.33	66.67	0.00	100.00	16.67
Min, Max	100.0, 100.0	0.0, 50.0	50.0, 83.3	-16.7, 33.3	50.0, 100.0	-16.7, 50.0

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Modified Randomized Set

Role Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	100.00 (NA)	0.00 (NA)	100.00 (NA)	16.67 (NA)	100.00 (0.000)	8.33 (11.785)
Median	100.00	0.00	100.00	16.67	100.00	8.33
Min, Max	100.0, 100.0	0.0, 0.0	100.0, 100.0	16.7, 16.7	100.0, 100.0	0.0, 16.7

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Modified Randomized Set

Emotional Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		10		38	
Mean (SD)	77.08 (25.922)		75.83 (9.977)		76.75 (22.690)	
Median	83.33		83.33		83.33	
Min, Max	0.0, 100.0		58.3, 83.3		0.0, 100.0	
Week 2						
n	27	27	10	9		
Mean (SD)	83.33 (20.672)	6.48 (16.068)	68.33 (20.713)	-2.78 (13.819)		
Median	91.67	0.00	75.00	0.00		
Min, Max	33.3, 100.0	-25.0, 50.0	33.3, 91.7	-33.3, 16.7		

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Emotional Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	28	28	10	10		
Mean (SD)	81.25 (24.806)	4.17 (17.347)	75.83 (22.720)	0.00 (15.215)		
Median	91.67	0.00	83.33	0.00		
Min, Max	8.3, 100.0	-50.0, 33.3	33.3, 100.0	-33.3, 16.7		
Week 8						
n	22	22	7	7		
Mean (SD)	84.85 (17.563)	6.44 (17.046)	88.10 (15.853)	13.10 (6.557)		
Median	87.50	0.00	100.00	16.67		
Min, Max	41.7, 100.0	-25.0, 58.3	66.7, 100.0	0.0, 16.7		

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Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	25	25	10	10		
Mean (SD)	83.00 (22.371)	7.00 (12.426)	80.00 (17.656)	4.17 (15.836)		
Median	91.67	0.00	79.17	8.33		
Min, Max	16.7, 100.0	-8.3, 41.7	50.0, 100.0	-33.3, 16.7		
Week 24						
n	22	22	10	10		
Mean (SD)	83.33 (23.146)	8.71 (18.090)	86.67 (11.915)	10.83 (5.625)		
Median	91.67	0.00	87.50	8.33		
Min, Max	8.3, 100.0	-25.0, 66.7	66.7, 100.0	0.0, 16.7		

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Emotional Functioning

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Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	10	10	29	29
Mean (SD)	81.58 (22.666)	3.51 (15.791)	79.17 (16.782)	3.33 (13.147)	80.75 (20.546)	3.45 (14.692)
Median	91.67	8.33	70.83	4.17	83.33	8.33
Min, Max	16.7, 100.0	-33.3, 25.0	58.3, 100.0	-25.0, 16.7	16.7, 100.0	-33.3, 25.0
Week 56						
n	13	13	8	8	21	21
Mean (SD)	83.33 (23.323)	7.05 (20.083)	73.96 (18.601)	-3.13 (14.042)	79.76 (21.662)	3.17 (18.348)
Median	100.00	8.33	66.67	-4.17	83.33	0.00
Min, Max	33.3, 100.0	-41.7, 33.3	50.0, 100.0	-16.7, 16.7	33.3, 100.0	-41.7, 33.3

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Table GERMANY.2.5.11.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Emotional Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	6	6	14	14
Mean (SD)	91.67 (13.363)	3.13 (17.216)	83.33 (15.811)	8.33 (7.454)	88.10 (14.513)	5.36 (13.715)
Median	100.00	4.17	83.33	8.33	95.83	8.33
Min, Max	66.7, 100.0	-33.3, 25.0	66.7, 100.0	0.0, 16.7	66.7, 100.0	-33.3, 25.0
Week 88						
n	6	6	4	4	10	10
Mean (SD)	97.22 (4.303)	12.50 (8.740)	81.25 (21.916)	6.25 (18.478)	90.83 (15.441)	10.00 (12.910)
Median	100.00	12.50	87.50	8.33	95.83	12.50
Min, Max	91.7, 100.0	0.0, 25.0	50.0, 100.0	-16.7, 25.0	50.0, 100.0	-16.7, 25.0

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Modified Randomized Set

Emotional Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	100.00 (NA)	0.00 (NA)	33.33 (NA)	-33.33 (NA)	66.67 (47.140)	-16.67 (23.570)
Median	100.00	0.00	33.33	-33.33	66.67	-16.67
Min, Max	100.0, 100.0	0.0, 0.0	33.3, 33.3	-33.3, -33.3	33.3, 100.0	-33.3, 0.0

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Table GERMANY.2.5.11.9
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Modified Randomized Set

Cognitive Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		10		38	
Mean (SD)	77.98 (24.450)		76.67 (14.055)		77.63 (22.014)	
Median	83.33		66.67		83.33	
Min, Max	16.7, 100.0		66.7, 100.0		16.7, 100.0	
Week 2						
n	27	27	9	9		
Mean (SD)	82.10 (19.571)	4.32 (17.042)	77.78 (22.048)	3.70 (21.695)		
Median	83.33	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-16.7, 50.0	33.3, 100.0	-33.3, 33.3		

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Cognitive Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	28	28	10	10		
Mean (SD)	84.52 (17.526)	6.55 (17.177)	73.33 (21.082)	-3.33 (23.307)		
Median	83.33	0.00	75.00	-8.33		
Min, Max	33.3, 100.0	-33.3, 50.0	33.3, 100.0	-33.3, 33.3		
Week 8						
n	22	22	7	7		
Mean (SD)	84.85 (17.750)	8.33 (20.412)	80.95 (17.817)	7.14 (13.113)		
Median	83.33	0.00	83.33	16.67		
Min, Max	33.3, 100.0	-33.3, 66.7	50.0, 100.0	-16.7, 16.7		

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Modified Randomized Set

Cognitive Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	25	25	10	10		
Mean (SD)	84.00 (18.308)	7.33 (14.498)	83.33 (13.608)	6.67 (14.055)		
Median	83.33	0.00	83.33	0.00		
Min, Max	33.3, 100.0	-16.7, 33.3	66.7, 100.0	-16.7, 33.3		
Week 24						
n	22	22	10	10		
Mean (SD)	81.06 (25.352)	5.30 (18.819)	83.33 (13.608)	6.67 (11.653)		
Median	91.67	0.00	83.33	8.33		
Min, Max	0.0, 100.0	-33.3, 50.0	66.7, 100.0	-16.7, 16.7		

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Cognitive Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	10	10	29	29
Mean (SD)	78.07 (23.605)	3.51 (18.070)	75.00 (16.197)	-1.67 (14.593)	77.01 (21.088)	1.72 (16.871)
Median	83.33	0.00	66.67	0.00	83.33	0.00
Min, Max	33.3, 100.0	-50.0, 33.3	50.0, 100.0	-33.3, 16.7	33.3, 100.0	-50.0, 33.3
Week 56						
n	13	13	8	8	21	21
Mean (SD)	79.49 (23.721)	3.85 (18.199)	77.08 (17.678)	2.08 (13.909)	78.57 (21.176)	3.17 (16.346)
Median	83.33	0.00	75.00	0.00	83.33	0.00
Min, Max	33.3, 100.0	-33.3, 33.3	50.0, 100.0	-16.7, 16.7	33.3, 100.0	-33.3, 33.3

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Cognitive Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	5	5	13	13
Mean (SD)	87.50 (14.773)	2.08 (13.909)	83.33 (16.667)	6.67 (9.129)	85.90 (14.979)	3.85 (12.084)
Median	91.67	0.00	83.33	0.00	83.33	0.00
Min, Max	66.7, 100.0	-16.7, 33.3	66.7, 100.0	0.0, 16.7	66.7, 100.0	-16.7, 33.3
Week 88						
n	6	6	4	4	10	10
Mean (SD)	97.22 (6.804)	13.89 (16.387)	66.67 (23.570)	-12.50 (15.957)	85.00 (21.445)	3.33 (20.488)
Median	100.00	8.33	75.00	-8.33	91.67	0.00
Min, Max	83.3, 100.0	0.0, 33.3	33.3, 83.3	-33.3, 0.0	33.3, 100.0	-33.3, 33.3

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Modified Randomized Set

Cognitive Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	100.00 (NA)	0.00 (NA)	33.33 (NA)	-33.33 (NA)	66.67 (47.140)	-16.67 (23.570)
Median	100.00	0.00	33.33	-33.33	66.67	-16.67
Min, Max	100.0, 100.0	0.0, 0.0	33.3, 33.3	-33.3, -33.3	33.3, 100.0	-33.3, 0.0

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Modified Randomized Set

Social Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		10		38	
Mean (SD)	76.19 (24.186)		63.33 (23.307)		72.81 (24.329)	
Median	83.33		66.67		75.00	
Min, Max	16.7, 100.0		16.7, 100.0		16.7, 100.0	
Week 2						
n	28	28	10	9		
Mean (SD)	80.95 (21.619)	4.76 (21.687)	66.67 (23.570)	3.70 (20.031)		
Median	91.67	0.00	66.67	0.00		
Min, Max	50.0, 100.0	-50.0, 66.7	16.7, 100.0	-16.7, 50.0		

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Social Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	28	28	10	10		
Mean (SD)	82.14 (21.721)	5.95 (15.190)	60.00 (17.916)	-3.33 (13.147)		
Median	91.67	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 33.3	16.7, 83.3	-16.7, 16.7		
Week 8						
n	22	22	7	7		
Mean (SD)	86.36 (20.339)	8.33 (26.602)	69.05 (27.936)	9.52 (16.265)		
Median	100.00	8.33	66.67	16.67		
Min, Max	33.3, 100.0	-50.0, 83.3	16.7, 100.0	-16.7, 33.3		

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Table GERMANY.2.5.11.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Social Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	25	25	10	10		
Mean (SD)	85.33 (20.593)	10.00 (19.837)	68.33 (16.574)	5.00 (17.656)		
Median	100.00	0.00	66.67	0.00		
Min, Max	33.3, 100.0	-33.3, 50.0	50.0, 100.0	-16.7, 33.3		
Week 24						
n	22	22	10	10		
Mean (SD)	79.55 (28.606)	3.79 (21.164)	76.67 (11.653)	13.33 (21.943)		
Median	100.00	0.00	75.00	16.67		
Min, Max	0.0, 100.0	-33.3, 33.3	66.7, 100.0	-16.7, 50.0		

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Social Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	10	10	29	29
Mean (SD)	73.68 (29.563)	-6.14 (24.976)	65.00 (28.814)	1.67 (38.849)	70.69 (29.092)	-3.45 (30.006)
Median	83.33	0.00	66.67	16.67	66.67	0.00
Min, Max	0.0, 100.0	-66.7, 33.3	0.0, 100.0	-100.0, 33.3	0.0, 100.0	-100.0, 33.3
Week 56						
n	13	13	8	8	21	21
Mean (SD)	85.90 (21.350)	3.85 (13.868)	75.00 (12.599)	4.17 (23.146)	81.75 (18.933)	3.97 (17.404)
Median	100.00	0.00	66.67	0.00	83.33	0.00
Min, Max	33.3, 100.0	-33.3, 16.7	66.7, 100.0	-16.7, 50.0	33.3, 100.0	-33.3, 50.0

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Modified Randomized Set

Social Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	8	8	6	6	14	14
Mean (SD)	85.42 (24.296)	4.17 (11.785)	77.78 (13.608)	5.56 (13.608)	82.14 (20.111)	4.76 (12.105)
Median	100.00	0.00	75.00	8.33	83.33	0.00
Min, Max	33.3, 100.0	-16.7, 16.7	66.7, 100.0	-16.7, 16.7	33.3, 100.0	-16.7, 16.7
Week 88						
n	6	6	4	4	10	10
Mean (SD)	100.00 (0.000)	11.11 (8.607)	75.00 (16.667)	0.00 (23.570)	90.00 (16.102)	6.67 (16.102)
Median	100.00	16.67	66.67	8.33	100.00	16.67
Min, Max	100.0, 100.0	0.0, 16.7	66.7, 100.0	-33.3, 16.7	66.7, 100.0	-33.3, 16.7

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Modified Randomized Set

Social Functioning

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	100.00 (NA)	0.00 (NA)	100.00 (NA)	50.00 (NA)	100.00 (0.000)	25.00 (35.355)
Median	100.00	0.00	100.00	50.00	100.00	25.00
Min, Max	100.0, 100.0	0.0, 0.0	100.0, 100.0	50.0, 50.0	100.0, 100.0	0.0, 50.0

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Modified Randomized Set

Global Health Status/QoL

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		4		11	
Mean (SD)	50.00 (15.957)		47.92 (14.232)		49.24 (14.651)	
Median	50.00		45.83		50.00	
Min, Max	25.0, 66.7		33.3, 66.7		25.0, 66.7	
Week 2						
n	7	7	5	4		
Mean (SD)	54.76 (19.159)	4.76 (27.995)	56.67 (19.003)	2.08 (10.486)		
Median	66.67	8.33	50.00	0.00		
Min, Max	16.7, 66.7	-50.0, 41.7	33.3, 83.3	-8.3, 16.7		

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Global Health Status/QoL

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	7	7	5	4		
Mean (SD)	61.90 (21.440)	11.90 (20.331)	53.33 (21.731)	-2.08 (4.167)		
Median	66.67	0.00	50.00	0.00		
Min, Max	25.0, 83.3	-8.3, 50.0	33.3, 83.3	-8.3, 0.0		
Week 8						
n	5	5	2	1		
Mean (SD)	60.00 (29.698)	11.67 (33.644)	50.00 (47.140)	-33.33 (NA)		
Median	66.67	16.67	50.00	-33.33		
Min, Max	25.0, 91.7	-41.7, 50.0	16.7, 83.3	-33.3, -33.3		

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Global Health Status/QoL

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	7	7	5	4		
Mean (SD)	67.86 (10.125)	17.86 (11.210)	51.67 (19.003)	-2.08 (14.232)		
Median	66.67	16.67	50.00	-4.17		
Min, Max	50.0, 83.3	0.0, 33.3	33.3, 75.0	-16.7, 16.7		
Week 24						
n	7	7	5	4		
Mean (SD)	65.48 (15.537)	15.48 (13.968)	60.00 (14.907)	10.42 (12.500)		
Median	66.67	16.67	66.67	8.33		
Min, Max	33.3, 83.3	0.0, 41.7	33.3, 66.7	0.0, 25.0		

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Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	7	7	4	4	11	11
Mean (SD)	59.52 (13.113)	9.52 (15.537)	50.00 (26.352)	2.08 (21.916)	56.06 (18.292)	6.82 (17.408)
Median	50.00	0.00	54.17	-4.17	50.00	0.00
Min, Max	50.0, 83.3	-8.3, 33.3	16.7, 75.0	-16.7, 33.3	16.7, 83.3	-16.7, 33.3
Week 56						
n	5	5	4	3	9	8
Mean (SD)	56.67 (23.124)	10.00 (22.361)	66.67 (19.245)	13.89 (29.266)	61.11 (20.833)	11.46 (23.119)
Median	50.00	0.00	66.67	16.67	50.00	8.33
Min, Max	33.3, 91.7	-8.3, 41.7	50.0, 83.3	-16.7, 41.7	33.3, 91.7	-16.7, 41.7

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Global Health Status/QoL

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	3	3	3	2	6	5
Mean (SD)	66.67 (0.000)	16.67 (22.048)	63.89 (29.266)	4.17 (29.463)	65.28 (18.572)	11.67 (22.515)
Median	66.67	8.33	66.67	4.17	66.67	8.33
Min, Max	66.7, 66.7	0.0, 41.7	33.3, 91.7	-16.7, 25.0	33.3, 91.7	-16.7, 41.7
Week 88						
n	1	1	2	2	3	3
Mean (SD)	58.33 (NA)	0.00 (NA)	50.00 (47.140)	4.17 (53.033)	52.78 (33.679)	2.78 (37.577)
Median	58.33	0.00	50.00	4.17	58.33	0.00
Min, Max	58.3, 58.3	0.0, 0.0	16.7, 83.3	-33.3, 41.7	16.7, 83.3	-33.3, 41.7

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Global Health Status/QoL

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	58.33 (NA)	0.00 (NA)	83.33 (NA)	41.67 (NA)	70.83 (17.678)	20.83 (29.463)
Median	58.33	0.00	83.33	41.67	70.83	20.83
Min, Max	58.3, 58.3	0.0, 0.0	83.3, 83.3	41.7, 41.7	58.3, 83.3	0.0, 41.7

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Physical Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		4		11	
Mean (SD)	64.76 (21.676)		53.33 (24.944)		60.61 (22.401)	
Median	66.67		50.00		60.00	
Min, Max	33.3, 86.7		26.7, 86.7		26.7, 86.7	
Week 2						
n	7	7	5	4		
Mean (SD)	78.10 (15.258)	13.33 (18.856)	57.33 (18.012)	-1.67 (15.753)		
Median	86.67	6.67	46.67	-6.67		
Min, Max	53.3, 93.3	-6.7, 46.7	40.0, 80.0	-13.3, 20.0		

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Physical Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	7	7	5	4		
Mean (SD)	84.76 (15.736)	20.00 (18.459)	50.67 (23.851)	-11.67 (17.533)		
Median	86.67	13.33	46.67	-13.33		
Min, Max	60.0, 100.0	6.7, 60.0	26.7, 86.7	-26.7, 6.7		
Week 8						
n	5	5	2	1		
Mean (SD)	84.00 (19.206)	18.67 (8.692)	53.33 (47.140)	-26.67 (NA)		
Median	93.33	13.33	53.33	-26.67		
Min, Max	60.0, 100.0	13.3, 33.3	20.0, 86.7	-26.7, -26.7		

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Physical Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	7	7	5	4		
Mean (SD)	80.95 (12.430)	16.19 (13.801)	46.67 (22.608)	-15.00 (23.960)		
Median	86.67	13.33	46.67	-23.33		
Min, Max	66.7, 100.0	0.0, 40.0	20.0, 80.0	-33.3, 20.0		
Week 24						
n	7	7	5	4		
Mean (SD)	80.00 (19.245)	15.24 (30.237)	62.67 (22.410)	3.33 (8.607)		
Median	86.67	13.33	53.33	3.33		
Min, Max	53.3, 100.0	-33.3, 60.0	40.0, 86.7	-6.7, 13.3		

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Physical Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	7	7	4	4	11	11
Mean (SD)	83.81 (14.836)	19.05 (17.397)	56.67 (32.886)	3.33 (22.771)	73.94 (25.378)	13.33 (20.000)
Median	86.67	13.33	53.33	6.67	86.67	13.33
Min, Max	60.0, 100.0	0.0, 53.3	20.0, 100.0	-26.7, 26.7	20.0, 100.0	-26.7, 53.3
Week 56						
n	5	5	4	3	9	8
Mean (SD)	80.00 (19.437)	18.67 (26.415)	71.67 (14.782)	13.33 (29.059)	76.30 (17.033)	16.67 (25.448)
Median	86.67	13.33	73.33	26.67	80.00	20.00
Min, Max	53.3, 100.0	-6.7, 60.0	53.3, 86.7	-20.0, 33.3	53.3, 100.0	-20.0, 60.0

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Physical Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	3	3	3	2	6	5
Mean (SD)	82.22 (25.240)	22.22 (34.211)	55.56 (44.389)	-13.33 (37.712)	68.89 (35.444)	8.00 (36.332)
Median	93.33	13.33	66.67	-13.33	80.00	13.33
Min, Max	53.3, 100.0	-6.7, 60.0	6.7, 93.3	-40.0, 13.3	6.7, 100.0	-40.0, 60.0
Week 88						
n	1	1	2	2	3	3
Mean (SD)	66.67 (NA)	6.67 (NA)	60.00 (37.712)	10.00 (32.998)	62.22 (26.943)	8.89 (23.413)
Median	66.67	6.67	60.00	10.00	66.67	6.67
Min, Max	66.7, 66.7	6.7, 6.7	33.3, 86.7	-13.3, 33.3	33.3, 86.7	-13.3, 33.3

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Physical Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	73.33 (NA)	13.33 (NA)	86.67 (NA)	33.33 (NA)	80.00 (9.428)	23.33 (14.142)
Median	73.33	13.33	86.67	33.33	80.00	23.33
Min, Max	73.3, 73.3	13.3, 13.3	86.7, 86.7	33.3, 33.3	73.3, 86.7	13.3, 33.3

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Role Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		4		11	
Mean (SD)	61.90 (35.635)		45.83 (36.956)		56.06 (35.176)	
Median	66.67		33.33		66.67	
Min, Max	0.0, 100.0		16.7, 100.0		0.0, 100.0	
Week 2						
n	7	7	4	3		
Mean (SD)	69.05 (27.936)	7.14 (33.134)	66.67 (36.004)	11.11 (19.245)		
Median	66.67	0.00	75.00	0.00		
Min, Max	33.3, 100.0	-33.3, 66.7	16.7, 100.0	0.0, 33.3		

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Role Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	7	7	4	3		
Mean (SD)	69.05 (33.923)	7.14 (44.987)	54.17 (36.956)	-11.11 (25.459)		
Median	66.67	0.00	50.00	-16.67		
Min, Max	0.0, 100.0	-66.7, 83.3	16.7, 100.0	-33.3, 16.7		
Week 8						
n	5	5	1	0		
Mean (SD)	80.00 (29.814)	13.33 (18.257)	100.00 (NA)			
Median	100.00	0.00	100.00			
Min, Max	33.3, 100.0	0.0, 33.3	100.0, 100.0			

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Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	7	7	4	3		
Mean (SD)	73.81 (26.972)	11.90 (20.893)	33.33 (27.217)	-27.78 (34.694)		
Median	66.67	0.00	33.33	-16.67		
Min, Max	33.3, 100.0	-16.7, 33.3	0.0, 66.7	-66.7, 0.0		
Week 24						
n	7	7	5	4		
Mean (SD)	59.52 (42.879)	-2.38 (45.571)	40.00 (38.370)	-12.50 (68.550)		
Median	66.67	0.00	50.00	0.00		
Min, Max	0.0, 100.0	-66.7, 83.3	0.0, 83.3	-100.0, 50.0		

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Role Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	7	7	4	4	11	11
Mean (SD)	76.19 (16.265)	14.29 (26.227)	25.00 (28.868)	-20.83 (36.956)	57.58 (32.799)	1.52 (33.710)
Median	66.67	0.00	25.00	-33.33	66.67	0.00
Min, Max	66.7, 100.0	0.0, 66.7	0.0, 50.0	-50.0, 33.3	0.0, 100.0	-50.0, 66.7
Week 56						
n	5	5	4	3	9	8
Mean (SD)	56.67 (14.907)	3.33 (41.500)	66.67 (0.000)	16.67 (44.096)	61.11 (11.785)	8.33 (39.841)
Median	66.67	0.00	66.67	33.33	66.67	8.33
Min, Max	33.3, 66.7	-33.3, 66.7	66.7, 66.7	-33.3, 50.0	33.3, 66.7	-33.3, 66.7

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Modified Randomized Set

Role Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	3	3	2	1	5	4
Mean (SD)	72.22 (25.459)	27.78 (34.694)	58.33 (11.785)	33.33 (NA)	66.67 (20.412)	29.17 (28.464)
Median	66.67	16.67	58.33	33.33	66.67	25.00
Min, Max	50.0, 100.0	0.0, 66.7	50.0, 66.7	33.3, 33.3	50.0, 100.0	0.0, 66.7
Week 88						
n	1	1	2	2	3	3
Mean (SD)	50.00 (NA)	16.67 (NA)	50.00 (47.140)	25.00 (58.926)	50.00 (33.333)	22.22 (41.944)
Median	50.00	16.67	50.00	25.00	50.00	16.67
Min, Max	50.0, 50.0	16.7, 16.7	16.7, 83.3	-16.7, 66.7	16.7, 83.3	-16.7, 66.7

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Modified Randomized Set

Role Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	50.00 (NA)	16.67 (NA)	66.67 (NA)	50.00 (NA)	58.33 (11.785)	33.33 (23.570)
Median	50.00	16.67	66.67	50.00	58.33	33.33
Min, Max	50.0, 50.0	16.7, 16.7	66.7, 66.7	50.0, 50.0	50.0, 66.7	16.7, 50.0

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Modified Randomized Set

Emotional Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		4		11	
Mean (SD)	76.19 (22.272)		64.58 (44.292)		71.97 (30.339)	
Median	75.00		75.00		75.00	
Min, Max	41.7, 100.0		8.3, 100.0		8.3, 100.0	
Week 2						
n	7	7	5	4		
Mean (SD)	78.57 (18.545)	2.38 (16.467)	75.00 (27.639)	4.17 (22.048)		
Median	75.00	0.00	75.00	8.33		
Min, Max	58.3, 100.0	-16.7, 33.3	33.3, 100.0	-25.0, 25.0		

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Modified Randomized Set

Emotional Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	7	7	5	4		
Mean (SD)	76.19 (34.166)	0.00 (34.021)	76.67 (24.580)	6.25 (29.950)		
Median	83.33	0.00	75.00	12.50		
Min, Max	0.0, 100.0	-66.7, 41.7	41.7, 100.0	-33.3, 33.3		
Week 8						
n	5	5	2	1		
Mean (SD)	80.00 (18.257)	8.33 (22.822)	91.67 (11.785)	-16.67 (NA)		
Median	66.67	8.33	91.67	-16.67		
Min, Max	66.7, 100.0	-25.0, 33.3	83.3, 100.0	-16.7, -16.7		

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Emotional Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	7	7	5	4		
Mean (SD)	83.33 (14.434)	7.14 (13.113)	55.00 (34.661)	-14.58 (24.884)		
Median	83.33	8.33	58.33	-8.33		
Min, Max	66.7, 100.0	-8.3, 33.3	0.0, 91.7	-50.0, 8.3		
Week 24						
n	7	7	5	4		
Mean (SD)	70.24 (34.647)	-5.95 (29.152)	81.67 (16.029)	18.75 (27.534)		
Median	66.67	0.00	83.33	16.67		
Min, Max	0.0, 100.0	-66.7, 25.0	58.3, 100.0	-8.3, 50.0		

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Emotional Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	7	7	4	4	11	11
Mean (SD)	69.05 (32.884)	-7.14 (30.968)	79.17 (8.333)	14.58 (40.469)	72.73 (26.376)	0.76 (34.451)
Median	75.00	-8.33	75.00	8.33	75.00	-8.33
Min, Max	0.0, 100.0	-66.7, 33.3	75.0, 91.7	-25.0, 66.7	0.0, 100.0	-66.7, 66.7
Week 56						
n	5	5	4	3	9	8
Mean (SD)	58.33 (36.324)	-10.00 (35.059)	91.67 (6.804)	38.89 (37.577)	73.15 (31.396)	8.33 (41.786)
Median	66.67	0.00	91.67	41.67	83.33	4.17
Min, Max	0.0, 100.0	-66.7, 25.0	83.3, 100.0	0.0, 75.0	0.0, 100.0	-66.7, 75.0

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Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	3	3	3	2	6	5
Mean (SD)	77.78 (20.972)	5.56 (25.459)	72.22 (29.266)	4.17 (41.248)	75.00 (22.973)	5.00 (27.386)
Median	75.00	0.00	75.00	4.17	75.00	0.00
Min, Max	58.3, 100.0	-16.7, 33.3	41.7, 100.0	-25.0, 33.3	41.7, 100.0	-25.0, 33.3
Week 88						
n	1	1	2	2	3	3
Mean (SD)	66.67 (NA)	-8.33 (NA)	79.17 (17.678)	25.00 (82.496)	75.00 (14.434)	13.89 (61.426)
Median	66.67	-8.33	79.17	25.00	66.67	-8.33
Min, Max	66.7, 66.7	-8.3, -8.3	66.7, 91.7	-33.3, 83.3	66.7, 91.7	-33.3, 83.3

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Emotional Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	83.33 (NA)	8.33 (NA)	91.67 (NA)	83.33 (NA)	87.50 (5.893)	45.83 (53.033)
Median	83.33	8.33	91.67	83.33	87.50	45.83
Min, Max	83.3, 83.3	8.3, 8.3	91.7, 91.7	83.3, 83.3	83.3, 91.7	8.3, 83.3

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Modified Randomized Set

Cognitive Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		4		11	
Mean (SD)	66.67 (25.459)		62.50 (28.464)		65.15 (25.226)	
Median	66.67		58.33		66.67	
Min, Max	16.7, 100.0		33.3, 100.0		16.7, 100.0	
Week 2						
n	7	7	5	4		
Mean (SD)	83.33 (19.245)	16.67 (27.217)	56.67 (38.370)	-12.50 (20.972)		
Median	83.33	16.67	83.33	-16.67		
Min, Max	50.0, 100.0	-16.7, 66.7	0.0, 83.3	-33.3, 16.7		

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Cognitive Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	7	7	5	4		
Mean (SD)	73.81 (26.972)	7.14 (30.211)	53.33 (39.791)	-16.67 (13.608)		
Median	83.33	16.67	50.00	-16.67		
Min, Max	16.7, 100.0	-50.0, 50.0	0.0, 100.0	-33.3, 0.0		
Week 8						
n	5	5	2	1		
Mean (SD)	76.67 (19.003)	13.33 (13.944)	58.33 (35.355)	0.00 (NA)		
Median	83.33	16.67	58.33	0.00		
Min, Max	50.0, 100.0	0.0, 33.3	33.3, 83.3	0.0, 0.0		

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1
and Period 2 and Long Term Extension by Race
Modified Randomized Set

Cognitive Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	7	7	5	4		
Mean (SD)	80.95 (14.996)	14.29 (20.250)	50.00 (31.180)	-20.83 (8.333)		
Median	83.33	0.00	33.33	-16.67		
Min, Max	66.7, 100.0	0.0, 50.0	16.7, 83.3	-33.3, -16.7		
Week 24						
n	7	7	5	4		
Mean (SD)	73.81 (34.503)	7.14 (37.090)	66.67 (31.180)	0.00 (23.570)		
Median	83.33	16.67	83.33	-8.33		
Min, Max	0.0, 100.0	-66.7, 50.0	33.3, 100.0	-16.7, 33.3		

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Modified Randomized Set

Cognitive Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	7	7	4	4	11	11
Mean (SD)	71.43 (32.934)	4.76 (35.635)	54.17 (45.896)	-8.33 (28.868)	65.15 (36.859)	0.00 (32.489)
Median	83.33	16.67	58.33	-16.67	83.33	0.00
Min, Max	0.0, 100.0	-66.7, 50.0	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-66.7, 50.0
Week 56						
n	5	5	4	3	9	8
Mean (SD)	73.33 (34.561)	10.00 (38.370)	66.67 (45.134)	-11.11 (41.944)	70.37 (37.060)	2.08 (38.253)
Median	83.33	16.67	83.33	-16.67	83.33	8.33
Min, Max	16.7, 100.0	-50.0, 50.0	0.0, 100.0	-50.0, 33.3	0.0, 100.0	-50.0, 50.0

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Modified Randomized Set

Cognitive Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	3	3	3	2	6	5
Mean (SD)	88.89 (19.245)	27.78 (25.459)	66.67 (44.096)	0.00 (23.570)	77.78 (32.773)	16.67 (26.352)
Median	100.00	33.33	83.33	0.00	91.67	16.67
Min, Max	66.7, 100.0	0.0, 50.0	16.7, 100.0	-16.7, 16.7	16.7, 100.0	-16.7, 50.0
Week 88						
n	1	1	2	2	3	3
Mean (SD)	83.33 (NA)	16.67 (NA)	50.00 (70.711)	0.00 (47.140)	61.11 (53.576)	5.56 (34.694)
Median	83.33	16.67	50.00	0.00	83.33	16.67
Min, Max	83.3, 83.3	16.7, 16.7	0.0, 100.0	-33.3, 33.3	0.0, 100.0	-33.3, 33.3

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Modified Randomized Set

Cognitive Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	66.67 (NA)	0.00 (NA)	83.33 (NA)	16.67 (NA)	75.00 (11.785)	8.33 (11.785)
Median	66.67	0.00	83.33	16.67	75.00	8.33
Min, Max	66.7, 66.7	0.0, 0.0	83.3, 83.3	16.7, 16.7	66.7, 83.3	0.0, 16.7

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Modified Randomized Set

Social Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	7		4		11	
Mean (SD)	71.43 (35.635)		50.00 (57.735)		63.64 (43.345)	
Median	66.67		50.00		66.67	
Min, Max	0.0, 100.0		0.0, 100.0		0.0, 100.0	
Week 2						
n	7	7	4	3		
Mean (SD)	80.95 (20.250)	9.52 (26.972)	45.83 (45.896)	-5.56 (25.459)		
Median	83.33	0.00	41.67	0.00		
Min, Max	50.0, 100.0	-33.3, 50.0	0.0, 100.0	-33.3, 16.7		

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Modified Randomized Set

Social Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 4						
n	7	7	4	3		
Mean (SD)	76.19 (25.198)	4.76 (35.635)	50.00 (40.825)	0.00 (50.000)		
Median	66.67	0.00	50.00	0.00		
Min, Max	33.3, 100.0	-33.3, 66.7	0.0, 100.0	-50.0, 50.0		
Week 8						
n	5	5	1	0		
Mean (SD)	80.00 (29.814)	20.00 (18.257)	100.00 (NA)			
Median	100.00	33.33	100.00			
Min, Max	33.3, 100.0	0.0, 33.3	100.0, 100.0			

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Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 12						
n	7	7	5	4		
Mean (SD)	78.57 (24.934)	7.14 (18.898)	30.00 (41.500)	-37.50 (43.833)		
Median	83.33	0.00	16.67	-33.33		
Min, Max	33.3, 100.0	-16.7, 33.3	0.0, 100.0	-83.3, 0.0		
Week 24						
n	7	7	4	3		
Mean (SD)	69.05 (33.923)	-2.38 (43.492)	45.83 (45.896)	-5.56 (25.459)		
Median	66.67	0.00	41.67	0.00		
Min, Max	0.0, 100.0	-66.7, 66.7	0.0, 100.0	-33.3, 16.7		

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Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	7	7	4	4	11	11
Mean (SD)	80.95 (20.250)	9.52 (30.211)	12.50 (25.000)	-37.50 (75.000)	56.06 (40.327)	-7.58 (52.896)
Median	83.33	0.00	0.00	-50.00	66.67	0.00
Min, Max	50.0, 100.0	-16.7, 66.7	0.0, 50.0	-100.0, 50.0	0.0, 100.0	-100.0, 66.7
Week 56						
n	5	5	4	3	9	8
Mean (SD)	70.00 (24.721)	10.00 (40.139)	62.50 (28.464)	16.67 (60.093)	66.67 (25.000)	12.50 (44.320)
Median	66.67	0.00	58.33	33.33	66.67	16.67
Min, Max	33.3, 100.0	-33.3, 66.7	33.3, 100.0	-50.0, 66.7	33.3, 100.0	-50.0, 66.7

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Social Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 72						
n	3	3	2	1	5	4
Mean (SD)	77.78 (25.459)	22.22 (34.694)	58.33 (58.926)	-83.33 (NA)	70.00 (36.132)	-4.17 (59.900)
Median	83.33	33.33	58.33	-83.33	83.33	8.33
Min, Max	50.0, 100.0	-16.7, 50.0	16.7, 100.0	-83.3, -83.3	16.7, 100.0	-83.3, 50.0
Week 88						
n	1	1	2	2	3	3
Mean (SD)	83.33 (NA)	-16.67 (NA)	66.67 (0.000)	16.67 (70.711)	72.22 (9.623)	5.56 (53.576)
Median	83.33	-16.67	66.67	16.67	66.67	-16.67
Min, Max	83.3, 83.3	-16.7, -16.7	66.7, 66.7	-33.3, 66.7	66.7, 83.3	-33.3, 66.7

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.11.9
Summary of Actual Values and Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During Treatment Period 1 and Period 2 and Long Term Extension by Race
Modified Randomized Set

Social Functioning

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 104						
n	1	1	1	1	2	2
Mean (SD)	66.67 (NA)	-33.33 (NA)	66.67 (NA)	66.67 (NA)	66.67 (0.000)	16.67 (70.711)
Median	66.67	-33.33	66.67	66.67	66.67	16.67
Min, Max	66.7, 66.7	-33.3, -33.3	66.7, 66.7	66.7, 66.7	66.7, 66.7	-33.3, 66.7

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.2
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL
Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	10.91 (2.509)	8.46 (4.004)	2.44 (4.616)	0.6009
	95% CI for LS MEAN	(5.75, 16.06)	(0.25, 16.67)	(-7.04, 11.93)	
Week 4	LS Mean (SEM)	9.46 (3.040)	4.45 (4.698)	5.00 (5.536)	0.3740
	95% CI for LS MEAN	(3.24, 15.68)	(-5.18, 14.09)	(-6.35, 16.35)	
Week 8	LS Mean (SEM)	6.54 (3.357)	10.32 (5.375)	-3.78 (6.267)	0.5521
	95% CI for LS MEAN	(-0.37, 13.46)	(-0.75, 21.40)	(-16.71, 9.15)	
Week 12	LS Mean (SEM)	12.54 (2.935)	9.08 (4.472)	3.46 (5.289)	0.5187
	95% CI for LS MEAN	(6.54, 18.54)	(-0.08, 18.25)	(-7.38, 14.30)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.2
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL
Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	4.11 (2.947)	-1.45 (4.439)	5.55 (5.281)	0.2986
	95% CI for LS MEAN	(-1.83, 10.04)	(-10.38, 7.49)	(-5.08, 16.19)	
Week 4	LS Mean (SEM)	9.99 (2.994)	-4.78 (4.510)	14.77 (5.367)	0.0084
	95% CI for LS MEAN	(3.96, 16.01)	(-13.85, 4.29)	(3.97, 25.57)	
Week 8	LS Mean (SEM)	11.24 (4.138)	-9.15 (6.291)	20.39 (7.496)	0.0095
	95% CI for LS MEAN	(2.88, 19.59)	(-21.84, 3.54)	(5.25, 35.53)	
Week 12	LS Mean (SEM)	11.41 (2.942)	3.55 (4.310)	7.86 (5.167)	0.1366
	95% CI for LS MEAN	(5.47, 17.35)	(-5.17, 12.27)	(-2.60, 18.31)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.2
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL

Subgroup by treatment interaction p-value 0.2930

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.2
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning
Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	7.81 (2.533)	-0.82 (3.967)	8.63 (4.596)	0.0712
	95% CI for LS MEAN	(2.61, 13.01)	(-8.95, 7.30)	(-0.80, 18.06)	
Week 4	LS Mean (SEM)	12.06 (2.560)	-0.63 (3.915)	12.70 (4.585)	0.0101
	95% CI for LS MEAN	(6.81, 17.31)	(-8.67, 7.41)	(3.28, 22.11)	
Week 8	LS Mean (SEM)	8.24 (2.575)	1.18 (4.016)	7.06 (4.667)	0.1450
	95% CI for LS MEAN	(2.90, 13.59)	(-7.15, 9.51)	(-2.64, 16.76)	
Week 12	LS Mean (SEM)	10.06 (2.848)	-2.85 (4.357)	12.92 (5.121)	0.0177
	95% CI for LS MEAN	(4.24, 15.89)	(-11.78, 6.08)	(2.42, 23.41)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.2
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning
Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	4.69 (1.920)	-1.69 (2.869)	6.38 (3.425)	0.0691
	95% CI for LS MEAN	(0.82, 8.56)	(-7.47, 4.09)	(-0.52, 13.28)	
Week 4	LS Mean (SEM)	10.57 (2.557)	-2.14 (3.833)	12.71 (4.587)	0.0080
	95% CI for LS MEAN	(5.43, 15.72)	(-9.85, 5.57)	(3.48, 21.94)	
Week 8	LS Mean (SEM)	9.59 (3.003)	-3.42 (4.519)	13.01 (5.416)	0.0204
	95% CI for LS MEAN	(3.55, 15.64)	(-12.51, 5.67)	(2.11, 23.91)	
Week 12	LS Mean (SEM)	11.32 (2.546)	-1.69 (3.722)	13.01 (4.489)	0.0059
	95% CI for LS MEAN	(6.19, 16.45)	(-9.20, 5.82)	(3.96, 22.07)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.2
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning

Subgroup by treatment interaction p-value	0.8569
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.2
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning
Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.82 (4.722)	-0.30 (7.325)	6.12 (8.511)	0.4777
	95% CI for LS MEAN	(-3.85, 15.50)	(-15.28, 14.68)	(-11.31, 23.56)	
Week 4	LS Mean (SEM)	11.14 (4.709)	2.24 (7.156)	8.90 (8.378)	0.2974
	95% CI for LS MEAN	(1.50, 20.79)	(-12.43, 16.92)	(-8.28, 26.08)	
Week 8	LS Mean (SEM)	8.03 (4.939)	-9.49 (7.605)	17.52 (8.891)	0.0627
	95% CI for LS MEAN	(-2.24, 18.31)	(-25.34, 6.35)	(-1.02, 36.07)	
Week 12	LS Mean (SEM)	9.83 (5.071)	2.24 (7.711)	7.59 (9.056)	0.4094
	95% CI for LS MEAN	(-0.55, 20.22)	(-13.58, 18.07)	(-11.00, 26.18)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.2
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning
Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	10.25 (3.584)	2.52 (5.523)	7.73 (6.513)	0.2425
	95% CI for LS MEAN	(3.00, 17.50)	(-8.65, 13.69)	(-5.45, 20.91)	
Week 4	LS Mean (SEM)	16.14 (4.031)	-1.05 (6.275)	17.19 (7.384)	0.0248
	95% CI for LS MEAN	(8.01, 24.27)	(-13.70, 11.61)	(2.29, 32.08)	
Week 8	LS Mean (SEM)	16.41 (4.548)	-7.05 (7.196)	23.46 (8.437)	0.0089
	95% CI for LS MEAN	(7.16, 25.66)	(-21.66, 7.56)	(6.30, 40.62)	
Week 12	LS Mean (SEM)	16.60 (4.156)	2.52 (6.314)	14.08 (7.485)	0.0673
	95% CI for LS MEAN	(8.22, 24.99)	(-10.24, 15.29)	(-1.05, 29.21)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.2
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning

Subgroup by treatment interaction p-value 0.4935

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.2
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning
Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	3.22 (2.430)	0.36 (3.923)	2.86 (4.537)	0.5342
	95% CI for LS MEAN	(-1.76, 8.20)	(-7.67, 8.39)	(-6.45, 12.16)	
Week 4	LS Mean (SEM)	5.39 (2.129)	8.73 (3.276)	-3.34 (3.839)	0.3920
	95% CI for LS MEAN	(1.03, 9.75)	(2.02, 15.44)	(-11.21, 4.53)	
Week 8	LS Mean (SEM)	2.78 (2.387)	7.61 (3.782)	-4.83 (4.407)	0.2846
	95% CI for LS MEAN	(-2.15, 7.71)	(-0.21, 15.42)	(-13.95, 4.29)	
Week 12	LS Mean (SEM)	2.98 (3.057)	3.17 (4.751)	-0.19 (5.601)	0.9734
	95% CI for LS MEAN	(-3.28, 9.25)	(-6.60, 12.94)	(-11.70, 11.33)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.2
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning
Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.73 (2.443)	-0.14 (3.773)	6.88 (4.472)	0.1309
	95% CI for LS MEAN	(1.82, 11.65)	(-7.74, 7.45)	(-2.12, 15.88)	
Week 4	LS Mean (SEM)	4.49 (3.402)	-3.48 (5.205)	7.97 (6.200)	0.2050
	95% CI for LS MEAN	(-2.36, 11.33)	(-13.94, 6.98)	(-4.50, 20.43)	
Week 8	LS Mean (SEM)	8.36 (3.138)	0.70 (4.878)	7.66 (5.757)	0.1913
	95% CI for LS MEAN	(2.01, 14.71)	(-9.15, 10.55)	(-3.99, 19.31)	
Week 12	LS Mean (SEM)	10.41 (2.530)	-3.48 (3.772)	13.89 (4.508)	0.0036
	95% CI for LS MEAN	(5.31, 15.51)	(-11.08, 4.13)	(4.80, 22.98)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.2
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning

Subgroup by treatment interaction p-value	0.1651
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.2
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning
Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	2.67 (2.527)	0.92 (4.183)	1.75 (4.802)	0.7187
	95% CI for LS MEAN	(-2.50, 7.84)	(-7.62, 9.47)	(-8.09, 11.58)	
Week 4	LS Mean (SEM)	4.84 (2.657)	3.34 (4.168)	1.50 (4.888)	0.7608
	95% CI for LS MEAN	(-0.58, 10.27)	(-5.17, 11.86)	(-8.48, 11.49)	
Week 8	LS Mean (SEM)	2.27 (2.739)	4.51 (4.426)	-2.24 (5.136)	0.6663
	95% CI for LS MEAN	(-3.36, 7.90)	(-4.59, 13.61)	(-12.81, 8.33)	
Week 12	LS Mean (SEM)	3.43 (2.799)	1.49 (4.325)	1.94 (5.109)	0.7075
	95% CI for LS MEAN	(-2.30, 9.15)	(-7.36, 10.34)	(-8.52, 12.39)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.2
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning
Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.53 (3.284)	0.68 (5.068)	5.85 (6.093)	0.3417
	95% CI for LS MEAN	(-0.08, 13.14)	(-9.52, 10.87)	(-6.41, 18.11)	
Week 4	LS Mean (SEM)	7.09 (3.541)	-4.88 (5.470)	11.97 (6.560)	0.0741
	95% CI for LS MEAN	(-0.03, 14.21)	(-15.87, 6.11)	(-1.21, 25.15)	
Week 8	LS Mean (SEM)	10.82 (3.317)	-0.72 (5.175)	11.54 (6.184)	0.0684
	95% CI for LS MEAN	(4.13, 17.52)	(-11.13, 9.69)	(-0.91, 24.00)	
Week 12	LS Mean (SEM)	8.75 (3.137)	2.90 (4.765)	5.86 (5.743)	0.3136
	95% CI for LS MEAN	(2.43, 15.08)	(-6.71, 12.51)	(-5.73, 17.44)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.2
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning

Subgroup by treatment interaction p-value	0.2898
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.2
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning
Male

Visit	Statistics	Danicopan (N = 23)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	4.16 (3.970)	2.05 (6.271)	2.11 (7.308)	0.7744
	95% CI for LS MEAN	(-3.96, 12.29)	(-10.76, 14.86)	(-12.84, 17.07)	
Week 4	LS Mean (SEM)	7.79 (3.417)	4.53 (5.154)	3.26 (6.054)	0.5948
	95% CI for LS MEAN	(0.79, 14.78)	(-6.04, 15.10)	(-9.15, 15.67)	
Week 8	LS Mean (SEM)	3.10 (4.167)	-2.91 (6.496)	6.00 (7.615)	0.4398
	95% CI for LS MEAN	(-5.57, 11.76)	(-16.45, 10.63)	(-9.89, 21.89)	
Week 12	LS Mean (SEM)	5.79 (3.612)	2.68 (5.452)	3.12 (6.421)	0.6315
	95% CI for LS MEAN	(-1.61, 13.20)	(-8.52, 13.88)	(-10.07, 16.30)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.2
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning
Female

Visit	Statistics	Danicopan (N = 34)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	10.15 (2.977)	-4.45 (4.617)	14.60 (5.410)	0.0103
	95% CI for LS MEAN	(4.12, 16.18)	(-13.79, 4.89)	(3.65, 25.55)	
Week 4	LS Mean (SEM)	9.17 (3.276)	-4.43 (5.087)	13.60 (5.975)	0.0280
	95% CI for LS MEAN	(2.56, 15.79)	(-14.70, 5.83)	(1.54, 25.67)	
Week 8	LS Mean (SEM)	16.55 (3.914)	2.15 (6.181)	14.41 (7.225)	0.0548
	95% CI for LS MEAN	(8.58, 24.53)	(-10.42, 14.71)	(-0.31, 29.13)	
Week 12	LS Mean (SEM)	13.76 (3.758)	-9.01 (5.507)	22.77 (6.579)	0.0013
	95% CI for LS MEAN	(6.18, 21.34)	(-20.14, 2.12)	(9.47, 36.06)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.2
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Sex
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning

Subgroup by treatment interaction p-value 0.0726

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional sex variable and sex by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.3
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL
< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	7.36 (2.550)	1.77 (3.835)	5.58 (4.491)	0.2192
	95% CI for LS MEAN	(2.25, 12.47)	(-5.91, 9.46)	(-3.42, 14.59)	
Week 4	LS Mean (SEM)	9.59 (2.665)	-1.00 (4.009)	10.60 (4.704)	0.0283
	95% CI for LS MEAN	(4.26, 14.93)	(-9.03, 7.03)	(1.17, 20.03)	
Week 8	LS Mean (SEM)	9.24 (3.528)	-1.97 (5.464)	11.21 (6.423)	0.0873
	95% CI for LS MEAN	(2.15, 16.33)	(-12.95, 9.00)	(-1.70, 24.13)	
Week 12	LS Mean (SEM)	12.78 (2.408)	6.40 (3.576)	6.37 (4.187)	0.1342
	95% CI for LS MEAN	(7.95, 17.61)	(-0.77, 13.58)	(-2.03, 14.78)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.3
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL
≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	4.55 (3.282)	1.91 (5.567)	2.63 (6.534)	0.6913
	95% CI for LS MEAN	(-2.35, 11.45)	(-9.70, 13.53)	(-11.02, 16.29)	
Week 4	LS Mean (SEM)	9.23 (3.066)	-3.50 (4.837)	12.73 (5.820)	0.0426
	95% CI for LS MEAN	(2.78, 15.69)	(-13.70, 6.71)	(0.47, 24.99)	
Week 8	LS Mean (SEM)	6.89 (4.944)	-4.64 (7.988)	11.53 (9.459)	0.2368
	95% CI for LS MEAN	(-3.40, 17.17)	(-21.29, 12.01)	(-8.17, 31.22)	
Week 12	LS Mean (SEM)	7.81 (4.417)	2.06 (6.722)	5.75 (8.111)	0.4884
	95% CI for LS MEAN	(-1.50, 17.12)	(-12.27, 16.38)	(-11.45, 22.96)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.3
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL

Subgroup by treatment interaction p-value	0.5831
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.3
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning
< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	8.04 (1.834)	-1.10 (2.733)	9.13 (3.202)	0.0062
	95% CI for LS MEAN	(4.36, 11.71)	(-6.58, 4.38)	(2.71, 15.56)	
Week 4	LS Mean (SEM)	11.82 (2.270)	-1.10 (3.404)	12.92 (4.018)	0.0022
	95% CI for LS MEAN	(7.28, 16.37)	(-7.91, 5.72)	(4.87, 20.97)	
Week 8	LS Mean (SEM)	12.44 (2.213)	-0.63 (3.366)	13.06 (3.953)	0.0019
	95% CI for LS MEAN	(7.98, 16.89)	(-7.40, 6.14)	(5.10, 21.02)	
Week 12	LS Mean (SEM)	12.77 (2.133)	-0.36 (3.161)	13.12 (3.734)	0.0009
	95% CI for LS MEAN	(8.49, 17.04)	(-6.69, 5.98)	(5.64, 20.60)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.3
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning
≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	1.13 (2.676)	-3.62 (4.485)	4.75 (5.322)	0.3835
	95% CI for LS MEAN	(-4.51, 6.78)	(-13.01, 5.78)	(-6.43, 15.94)	
Week 4	LS Mean (SEM)	9.47 (3.313)	-3.30 (5.275)	12.77 (6.354)	0.0618
	95% CI for LS MEAN	(2.44, 16.50)	(-14.52, 7.92)	(-0.71, 26.25)	
Week 8	LS Mean (SEM)	1.93 (3.734)	-3.04 (5.867)	4.97 (7.051)	0.4897
	95% CI for LS MEAN	(-5.88, 9.74)	(-15.36, 9.28)	(-9.81, 19.75)	
Week 12	LS Mean (SEM)	4.68 (3.668)	-7.75 (5.731)	12.42 (6.917)	0.0890
	95% CI for LS MEAN	(-3.00, 12.35)	(-19.80, 4.31)	(-2.09, 26.93)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.3
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning

Subgroup by treatment interaction p-value	0.5872
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.3
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning
< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	11.09 (3.549)	2.18 (5.303)	8.92 (6.226)	0.1581
	95% CI for LS MEAN	(3.98, 18.21)	(-8.46, 12.81)	(-3.58, 21.41)	
Week 4	LS Mean (SEM)	15.84 (3.886)	1.25 (5.850)	14.59 (6.874)	0.0385
	95% CI for LS MEAN	(8.05, 23.62)	(-10.47, 12.97)	(0.80, 28.37)	
Week 8	LS Mean (SEM)	17.91 (3.988)	-6.83 (6.146)	24.74 (7.181)	0.0014
	95% CI for LS MEAN	(9.85, 25.97)	(-19.24, 5.57)	(10.23, 39.26)	
Week 12	LS Mean (SEM)	16.49 (3.847)	4.96 (5.738)	11.53 (6.757)	0.0935
	95% CI for LS MEAN	(8.79, 24.19)	(-6.54, 16.45)	(-2.01, 25.08)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.3
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning
≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.54 (5.435)	1.15 (9.630)	-1.69 (11.175)	0.8819
	95% CI for LS MEAN	(-12.07, 11.00)	(-19.24, 21.55)	(-25.35, 21.97)	
Week 4	LS Mean (SEM)	6.99 (4.517)	-0.41 (7.609)	7.40 (8.991)	0.4227
	95% CI for LS MEAN	(-2.59, 16.56)	(-16.58, 15.75)	(-11.68, 26.48)	
Week 8	LS Mean (SEM)	0.38 (5.051)	-7.16 (8.499)	7.54 (10.030)	0.4630
	95% CI for LS MEAN	(-10.26, 11.02)	(-25.19, 10.88)	(-13.68, 28.75)	
Week 12	LS Mean (SEM)	3.12 (5.148)	-3.75 (8.218)	6.87 (9.848)	0.4977
	95% CI for LS MEAN	(-7.89, 14.13)	(-21.58, 14.08)	(-14.36, 28.09)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.3
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning

Subgroup by treatment interaction p-value 0.3720

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.3
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning
< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	7.77 (2.090)	1.38 (3.188)	6.38 (3.720)	0.0919
	95% CI for LS MEAN	(3.58, 11.96)	(-5.01, 7.77)	(-1.08, 13.85)	
Week 4	LS Mean (SEM)	4.52 (2.880)	2.77 (4.372)	1.74 (5.169)	0.7371
	95% CI for LS MEAN	(-1.25, 10.28)	(-5.98, 11.52)	(-8.61, 12.09)	
Week 8	LS Mean (SEM)	7.98 (2.508)	5.88 (3.919)	2.09 (4.573)	0.6491
	95% CI for LS MEAN	(2.93, 13.03)	(-2.00, 13.76)	(-7.11, 11.30)	
Week 12	LS Mean (SEM)	8.62 (2.136)	1.85 (3.211)	6.77 (3.764)	0.0775
	95% CI for LS MEAN	(4.34, 12.90)	(-4.59, 8.28)	(-0.77, 14.32)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.3
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning
≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.22 (2.514)	-8.91 (4.518)	8.69 (5.209)	0.1131
	95% CI for LS MEAN	(-5.56, 5.13)	(-18.39, 0.58)	(-2.28, 19.65)	
Week 4	LS Mean (SEM)	6.78 (3.091)	-7.43 (5.058)	14.21 (5.957)	0.0292
	95% CI for LS MEAN	(0.25, 13.32)	(-18.12, 3.27)	(1.62, 26.79)	
Week 8	LS Mean (SEM)	3.46 (3.290)	-8.26 (5.276)	11.72 (6.249)	0.0785
	95% CI for LS MEAN	(-3.50, 10.41)	(-19.40, 2.89)	(-1.49, 24.92)	
Week 12	LS Mean (SEM)	4.60 (3.861)	-12.98 (6.198)	17.58 (7.329)	0.0282
	95% CI for LS MEAN	(-3.53, 12.73)	(-26.08, 0.12)	(2.12, 33.05)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning

Subgroup by treatment interaction p-value	0.6120
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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.3
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning
< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	7.67 (2.543)	3.76 (3.850)	3.91 (4.530)	0.3914
	95% CI for LS MEAN	(2.58, 12.76)	(-3.95, 11.47)	(-5.16, 12.99)	
Week 4	LS Mean (SEM)	7.67 (2.877)	0.98 (4.353)	6.69 (5.144)	0.1986
	95% CI for LS MEAN	(1.91, 13.43)	(-7.73, 9.69)	(-3.61, 16.99)	
Week 8	LS Mean (SEM)	9.54 (2.563)	1.82 (3.951)	7.71 (4.628)	0.1021
	95% CI for LS MEAN	(4.38, 14.69)	(-6.11, 9.76)	(-1.59, 17.02)	
Week 12	LS Mean (SEM)	9.63 (2.225)	4.68 (3.333)	4.95 (3.907)	0.2103
	95% CI for LS MEAN	(5.18, 14.09)	(-2.00, 11.36)	(-2.88, 12.79)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.3
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning
≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	0.43 (4.121)	-16.96 (6.455)	17.38 (7.741)	0.0450
	95% CI for LS MEAN	(-8.57, 9.43)	(-31.13, -2.78)	(0.46, 34.31)	
Week 4	LS Mean (SEM)	4.99 (4.248)	-17.33 (6.525)	22.31 (7.872)	0.0110
	95% CI for LS MEAN	(-3.92, 13.89)	(-31.08, -3.57)	(5.77, 38.86)	
Week 8	LS Mean (SEM)	0.73 (4.074)	-11.24 (6.193)	11.97 (7.455)	0.1248
	95% CI for LS MEAN	(-7.76, 9.22)	(-24.23, 1.74)	(-3.63, 27.57)	
Week 12	LS Mean (SEM)	4.20 (4.656)	-11.77 (6.750)	15.97 (8.280)	0.0742
	95% CI for LS MEAN	(-5.68, 14.08)	(-26.37, 2.82)	(-1.78, 33.72)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.3
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning

Subgroup by treatment interaction p-value	0.3871
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.3
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning
< 65 Years

Visit	Statistics	Danicopan (N = 41)	Placebo (N = 18)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	9.38 (3.152)	-1.81 (4.760)	11.20 (5.591)	0.0502
	95% CI for LS MEAN	(3.07, 15.70)	(-11.35, 7.72)	(-0.01, 22.40)	
Week 4	LS Mean (SEM)	9.38 (3.097)	-0.89 (4.677)	10.27 (5.489)	0.0667
	95% CI for LS MEAN	(3.18, 15.59)	(-10.26, 8.49)	(-0.73, 21.28)	
Week 8	LS Mean (SEM)	14.90 (3.369)	2.43 (5.188)	12.47 (6.070)	0.0459
	95% CI for LS MEAN	(8.11, 21.69)	(-8.02, 12.88)	(0.23, 24.71)	
Week 12	LS Mean (SEM)	12.94 (3.115)	-3.66 (4.649)	16.60 (5.475)	0.0037
	95% CI for LS MEAN	(6.70, 19.18)	(-12.98, 5.66)	(5.62, 27.58)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.3
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning
≥ 65 Years

Visit	Statistics	Danicopan (N = 16)	Placebo (N = 6)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	3.42 (5.954)	-11.37 (10.489)	14.79 (12.088)	0.2417
	95% CI for LS MEAN	(-9.40, 16.25)	(-33.84, 11.10)	(-11.18, 40.76)	
Week 4	LS Mean (SEM)	6.55 (5.063)	-6.44 (8.999)	12.99 (10.675)	0.3684
	95% CI for LS MEAN	(-23.69, 36.78)	(-54.09, 41.20)	(-43.10, 69.07)	
Week 8	LS Mean (SEM)	-0.05 (6.779)	-13.76 (12.938)	13.71 (14.865)	0.5116
	95% CI for LS MEAN	(-77.49, 77.39)	(-134.50, 106.99)	(-131.99, 159.41)	
Week 12	LS Mean (SEM)	4.52 (5.945)	-11.87 (10.181)	16.38 (11.818)	0.1822
	95% CI for LS MEAN	(-7.95, 16.99)	(-33.21, 9.48)	(-8.41, 41.18)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.3
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Age at Informed Consent (< 65 and ≥ 65 Years)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning

Subgroup by treatment interaction p-value	0.8607
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional age variable and age by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.4
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL
Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	10.31 (2.470)	-2.29 (3.656)	12.60 (4.378)	0.0069
	95% CI for LS MEAN	(5.29, 15.34)	(-9.72, 5.14)	(3.70, 21.50)	
Week 4	LS Mean (SEM)	10.31 (3.052)	-2.98 (4.479)	13.30 (5.393)	0.0188
	95% CI for LS MEAN	(4.11, 16.52)	(-12.08, 6.11)	(2.34, 24.25)	
Week 8	LS Mean (SEM)	10.55 (4.314)	-7.72 (6.698)	18.27 (7.952)	0.0283
	95% CI for LS MEAN	(1.74, 19.37)	(-21.34, 5.91)	(2.07, 34.47)	
Week 12	LS Mean (SEM)	14.80 (2.411)	-0.90 (3.475)	15.70 (4.197)	0.0008
	95% CI for LS MEAN	(9.87, 19.72)	(-8.01, 6.21)	(7.11, 24.28)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.4
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL
Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	2.53 (3.131)	7.86 (5.144)	-5.33 (5.922)	0.3738
	95% CI for LS MEAN	(-3.79, 8.86)	(-2.52, 18.24)	(-17.29, 6.64)	
Week 4	LS Mean (SEM)	7.74 (2.979)	1.79 (4.686)	5.95 (5.468)	0.2828
	95% CI for LS MEAN	(1.73, 13.76)	(-7.68, 11.26)	(-5.10, 17.00)	
Week 8	LS Mean (SEM)	6.06 (3.935)	5.43 (6.024)	0.63 (7.122)	0.9300
	95% CI for LS MEAN	(-1.89, 14.01)	(-6.77, 17.62)	(-13.80, 15.06)	
Week 12	LS Mean (SEM)	8.42 (3.033)	13.60 (4.664)	-5.17 (5.479)	0.3512
	95% CI for LS MEAN	(2.30, 14.55)	(4.15, 23.04)	(-16.26, 5.92)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.4
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL

Subgroup by treatment interaction p-value	0.0098
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.4
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning
Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.54 (2.095)	-3.65 (3.110)	9.19 (3.701)	0.0183
	95% CI for LS MEAN	(1.27, 9.81)	(-9.98, 2.68)	(1.66, 16.73)	
Week 4	LS Mean (SEM)	11.94 (2.604)	-7.54 (3.827)	19.48 (4.590)	0.0002
	95% CI for LS MEAN	(6.65, 17.23)	(-15.32, 0.23)	(10.15, 28.81)	
Week 8	LS Mean (SEM)	9.98 (3.273)	-12.21 (4.997)	22.19 (5.946)	0.0008
	95% CI for LS MEAN	(3.29, 16.67)	(-22.38, -2.05)	(10.07, 34.32)	
Week 12	LS Mean (SEM)	10.62 (2.980)	-8.10 (4.288)	18.72 (5.188)	0.0011
	95% CI for LS MEAN	(4.55, 16.69)	(-16.83, 0.64)	(8.14, 29.29)	

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Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.4
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning
Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.12 (2.196)	-0.01 (3.557)	6.13 (4.105)	0.1434
	95% CI for LS MEAN	(1.68, 10.57)	(-7.20, 7.18)	(-2.18, 14.45)	
Week 4	LS Mean (SEM)	10.25 (2.295)	3.81 (3.617)	6.44 (4.231)	0.1357
	95% CI for LS MEAN	(5.62, 14.88)	(-3.50, 11.12)	(-2.11, 15.00)	
Week 8	LS Mean (SEM)	7.93 (2.243)	6.06 (3.448)	1.87 (4.049)	0.6472
	95% CI for LS MEAN	(3.40, 12.46)	(-0.92, 13.04)	(-6.34, 10.07)	
Week 12	LS Mean (SEM)	10.48 (2.125)	3.25 (3.275)	7.23 (3.848)	0.0677
	95% CI for LS MEAN	(6.19, 14.77)	(-3.37, 9.88)	(-0.55, 15.01)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.4
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning

Subgroup by treatment interaction p-value	0.1084
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.4
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning
Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	9.04 (4.632)	1.20 (6.985)	7.84 (8.327)	0.3539
	95% CI for LS MEAN	(-0.41, 18.48)	(-13.04, 15.44)	(-9.14, 24.82)	
Week 4	LS Mean (SEM)	10.16 (5.756)	1.20 (8.755)	8.96 (10.420)	0.3960
	95% CI for LS MEAN	(-1.56, 21.88)	(-16.62, 19.02)	(-12.26, 30.18)	
Week 8	LS Mean (SEM)	15.68 (4.831)	-15.04 (8.270)	30.72 (9.512)	0.0036
	95% CI for LS MEAN	(5.63, 25.72)	(-32.09, 2.00)	(11.06, 50.37)	
Week 12	LS Mean (SEM)	12.91 (5.267)	-1.83 (7.880)	14.74 (9.410)	0.1270
	95% CI for LS MEAN	(2.19, 23.62)	(-17.88, 14.21)	(-4.42, 33.91)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.4
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning
Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	7.05 (3.954)	2.50 (6.521)	4.55 (7.507)	0.5483
	95% CI for LS MEAN	(-0.95, 15.06)	(-10.71, 15.72)	(-10.68, 19.78)	
Week 4	LS Mean (SEM)	16.42 (2.872)	0.82 (4.467)	15.61 (5.166)	0.0045
	95% CI for LS MEAN	(10.62, 22.23)	(-8.22, 9.86)	(5.15, 26.07)	
Week 8	LS Mean (SEM)	11.54 (3.976)	0.95 (6.169)	10.58 (7.230)	0.1521
	95% CI for LS MEAN	(3.50, 19.57)	(-11.56, 13.47)	(-4.09, 25.26)	
Week 12	LS Mean (SEM)	13.84 (3.587)	7.76 (5.551)	6.08 (6.494)	0.3551
	95% CI for LS MEAN	(6.60, 21.08)	(-3.47, 19.00)	(-7.07, 19.23)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.4

Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning

Subgroup by treatment interaction p-value	0.5543
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.4
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning
Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.65 (2.582)	-2.34 (3.821)	7.99 (4.594)	0.0913
	95% CI for LS MEAN	(0.39, 10.92)	(-10.11, 5.44)	(-1.36, 17.34)	
Week 4	LS Mean (SEM)	2.30 (4.222)	-0.95 (6.181)	3.25 (7.465)	0.6660
	95% CI for LS MEAN	(-6.28, 10.88)	(-13.49, 11.59)	(-11.91, 18.41)	
Week 8	LS Mean (SEM)	9.63 (2.725)	6.92 (4.394)	2.71 (5.118)	0.6016
	95% CI for LS MEAN	(3.97, 15.30)	(-2.13, 15.98)	(-7.87, 13.29)	
Week 12	LS Mean (SEM)	6.11 (2.993)	-5.12 (4.319)	11.23 (5.218)	0.0408
	95% CI for LS MEAN	(-0.04, 12.26)	(-13.98, 3.75)	(0.51, 21.95)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.4
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning
Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	4.64 (2.436)	2.43 (3.908)	2.21 (4.484)	0.6241
	95% CI for LS MEAN	(-0.28, 9.56)	(-5.46, 10.32)	(-6.85, 11.28)	
Week 4	LS Mean (SEM)	6.46 (2.315)	2.84 (3.602)	3.62 (4.166)	0.3899
	95% CI for LS MEAN	(1.79, 11.14)	(-4.44, 10.12)	(-4.80, 12.05)	
Week 8	LS Mean (SEM)	3.86 (2.980)	2.60 (4.601)	1.26 (5.385)	0.8167
	95% CI for LS MEAN	(-2.15, 9.87)	(-6.70, 11.91)	(-9.64, 12.15)	
Week 12	LS Mean (SEM)	7.74 (2.475)	2.84 (3.819)	4.89 (4.443)	0.2775
	95% CI for LS MEAN	(2.74, 12.73)	(-4.88, 10.57)	(-4.09, 13.88)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.4
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning

Subgroup by treatment interaction p-value 0.4350

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.4
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning
Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	3.90 (3.523)	-5.79 (5.203)	9.70 (6.268)	0.1312
	95% CI for LS MEAN	(-3.26, 11.07)	(-16.37, 4.78)	(-3.04, 22.44)	
Week 4	LS Mean (SEM)	2.44 (3.911)	-8.57 (5.788)	11.01 (6.969)	0.1230
	95% CI for LS MEAN	(-5.51, 10.39)	(-20.32, 3.17)	(-3.14, 25.16)	
Week 8	LS Mean (SEM)	5.90 (3.096)	0.18 (4.922)	5.72 (5.786)	0.3299
	95% CI for LS MEAN	(-0.42, 12.22)	(-9.83, 10.18)	(-6.06, 17.50)	
Week 12	LS Mean (SEM)	5.53 (3.411)	-1.63 (4.957)	7.15 (5.987)	0.2424
	95% CI for LS MEAN	(-1.47, 12.52)	(-11.78, 8.53)	(-5.12, 19.43)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

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Table GERMANY.2.5.10.4
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning
Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.62 (2.700)	6.72 (4.390)	-1.10 (5.041)	0.8285
	95% CI for LS MEAN	(0.17, 11.08)	(-2.13, 15.58)	(-11.28, 9.08)	
Week 4	LS Mean (SEM)	8.75 (2.644)	4.34 (4.138)	4.41 (4.810)	0.3645
	95% CI for LS MEAN	(3.41, 14.09)	(-4.03, 12.70)	(-5.31, 14.13)	
Week 8	LS Mean (SEM)	8.14 (2.908)	3.16 (4.470)	4.98 (5.230)	0.3466
	95% CI for LS MEAN	(2.28, 14.00)	(-5.86, 12.19)	(-5.59, 15.55)	
Week 12	LS Mean (SEM)	6.65 (2.727)	5.73 (4.152)	0.92 (4.868)	0.8509
	95% CI for LS MEAN	(1.15, 12.15)	(-2.67, 14.12)	(-8.92, 10.76)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.4

Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning

Subgroup by treatment interaction p-value	0.1782
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.4
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning
Europe

Visit	Statistics	Danicopan (N = 25)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	8.57 (3.960)	-9.43 (6.064)	18.00 (7.331)	0.0194
	95% CI for LS MEAN	(0.49, 16.65)	(-21.76, 2.90)	(3.09, 32.91)	
Week 4	LS Mean (SEM)	7.24 (3.734)	-11.76 (5.792)	19.00 (6.996)	0.0105
	95% CI for LS MEAN	(-0.39, 14.86)	(-23.55, 0.03)	(4.76, 33.24)	
Week 8	LS Mean (SEM)	10.03 (4.850)	-1.93 (8.169)	11.96 (9.538)	0.2219
	95% CI for LS MEAN	(-0.04, 20.10)	(-18.75, 14.89)	(-7.72, 31.63)	
Week 12	LS Mean (SEM)	8.93 (4.458)	-17.13 (6.513)	26.06 (7.946)	0.0024
	95% CI for LS MEAN	(-0.13, 17.99)	(-30.37, -3.90)	(9.92, 42.21)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:47:50

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.4
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning
Rest of World

Visit	Statistics	Danicopan (N = 32)	Placebo (N = 12)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.90 (3.228)	3.50 (5.204)	3.40 (6.033)	0.5763
	95% CI for LS MEAN	(0.37, 13.42)	(-7.02, 14.02)	(-8.81, 15.61)	
Week 4	LS Mean (SEM)	9.50 (3.069)	8.94 (4.869)	0.56 (5.652)	0.9217
	95% CI for LS MEAN	(3.30, 15.71)	(-0.91, 18.80)	(-10.89, 12.01)	
Week 8	LS Mean (SEM)	11.36 (3.260)	4.07 (4.997)	7.29 (5.857)	0.2218
	95% CI for LS MEAN	(4.76, 17.95)	(-6.08, 14.21)	(-4.61, 19.19)	
Week 12	LS Mean (SEM)	12.08 (2.685)	7.55 (4.106)	4.52 (4.785)	0.3504
	95% CI for LS MEAN	(6.65, 17.50)	(-0.76, 15.87)	(-5.16, 14.21)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:47:50

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.4
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Region
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning

Subgroup by treatment interaction p-value 0.0221

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional region variable and region by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.5
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL
> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.21 (3.653)	6.24 (5.866)	-0.03 (6.666)	0.9964
	95% CI for LS MEAN	(-1.23, 13.66)	(-5.70, 18.19)	(-13.64, 13.58)	
Week 4	LS Mean (SEM)	11.42 (2.759)	-4.58 (4.183)	16.00 (4.675)	0.0019
	95% CI for LS MEAN	(5.78, 17.06)	(-13.13, 3.97)	(6.44, 25.56)	
Week 8	LS Mean (SEM)	3.86 (4.654)	1.53 (7.742)	2.33 (8.856)	0.7941
	95% CI for LS MEAN	(-5.60, 13.32)	(-14.20, 17.27)	(-15.70, 20.37)	
Week 12	LS Mean (SEM)	9.12 (3.719)	11.16 (5.754)	-2.04 (6.612)	0.7598
	95% CI for LS MEAN	(1.50, 16.73)	(-0.67, 22.99)	(-15.67, 11.58)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.5
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL
≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.81 (2.617)	0.10 (3.883)	5.71 (4.682)	0.2290
	95% CI for LS MEAN	(0.54, 11.09)	(-7.73, 7.93)	(-3.73, 15.15)	
Week 4	LS Mean (SEM)	7.07 (3.058)	1.21 (4.537)	5.86 (5.470)	0.2896
	95% CI for LS MEAN	(0.91, 13.23)	(-7.93, 10.35)	(-5.15, 16.88)	
Week 8	LS Mean (SEM)	11.43 (3.533)	-3.52 (5.195)	14.95 (6.280)	0.0226
	95% CI for LS MEAN	(4.27, 18.59)	(-14.04, 7.01)	(2.22, 27.67)	
Week 12	LS Mean (SEM)	12.40 (2.513)	2.88 (3.625)	9.52 (4.408)	0.0364
	95% CI for LS MEAN	(7.34, 17.46)	(-4.44, 10.19)	(0.63, 18.41)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:47:52

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Table GERMANY.2.5.10.5

Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL

Subgroup by treatment interaction p-value	0.8624
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.5
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning
> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	2.69 (2.571)	2.50 (4.179)	0.18 (4.802)	0.9698
	95% CI for LS MEAN	(-2.55, 7.92)	(-5.98, 10.99)	(-9.59, 9.96)	
Week 4	LS Mean (SEM)	7.97 (2.310)	1.68 (3.623)	6.29 (4.185)	0.1434
	95% CI for LS MEAN	(3.25, 12.68)	(-5.71, 9.07)	(-2.26, 14.83)	
Week 8	LS Mean (SEM)	2.11 (2.823)	6.05 (4.641)	-3.94 (5.352)	0.4665
	95% CI for LS MEAN	(-3.64, 7.85)	(-3.39, 15.49)	(-14.85, 6.96)	
Week 12	LS Mean (SEM)	6.05 (2.483)	2.42 (3.847)	3.63 (4.492)	0.4255
	95% CI for LS MEAN	(0.97, 11.13)	(-5.47, 10.31)	(-5.57, 12.84)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.5
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning
≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	8.25 (1.818)	-3.13 (2.670)	11.38 (3.230)	0.0010
	95% CI for LS MEAN	(4.59, 11.92)	(-8.51, 2.26)	(4.87, 17.89)	
Week 4	LS Mean (SEM)	13.07 (2.712)	-2.68 (4.023)	15.76 (4.852)	0.0023
	95% CI for LS MEAN	(7.60, 18.55)	(-10.80, 5.43)	(5.97, 25.55)	
Week 8	LS Mean (SEM)	13.75 (2.647)	-5.53 (3.905)	19.28 (4.718)	0.0002
	95% CI for LS MEAN	(8.40, 19.11)	(-13.43, 2.37)	(9.74, 28.83)	
Week 12	LS Mean (SEM)	13.85 (2.559)	-4.02 (3.732)	17.87 (4.525)	0.0003
	95% CI for LS MEAN	(8.69, 19.02)	(-11.56, 3.52)	(8.73, 27.01)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.5

Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning

Subgroup by treatment interaction p-value	0.0463
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.5
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning
> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	3.83 (4.349)	12.80 (7.335)	-8.97 (7.948)	0.2692
	95% CI for LS MEAN	(-5.06, 12.72)	(-2.19, 27.79)	(-25.28, 7.34)	
Week 4	LS Mean (SEM)	12.86 (5.166)	2.94 (8.172)	9.92 (9.284)	0.2944
	95% CI for LS MEAN	(2.32, 23.40)	(-13.76, 19.64)	(-9.10, 28.94)	
Week 8	LS Mean (SEM)	8.70 (4.692)	4.48 (7.866)	4.22 (8.671)	0.6304
	95% CI for LS MEAN	(-0.88, 18.28)	(-11.59, 20.55)	(-13.57, 22.01)	
Week 12	LS Mean (SEM)	11.46 (5.053)	12.20 (7.836)	-0.74 (8.941)	0.9344
	95% CI for LS MEAN	(1.15, 21.77)	(-3.84, 28.24)	(-19.07, 17.59)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:47:52

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Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning
≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	11.46 (3.772)	-4.58 (5.635)	16.04 (6.781)	0.0229
	95% CI for LS MEAN	(3.84, 19.08)	(-15.97, 6.81)	(2.33, 29.74)	
Week 4	LS Mean (SEM)	14.59 (3.815)	0.18 (5.855)	14.41 (6.988)	0.0453
	95% CI for LS MEAN	(6.90, 22.29)	(-11.63, 12.00)	(0.31, 28.51)	
Week 8	LS Mean (SEM)	17.15 (4.241)	-14.88 (6.474)	32.03 (7.739)	0.0002
	95% CI for LS MEAN	(8.50, 25.79)	(-28.07, -1.69)	(16.26, 47.80)	
Week 12	LS Mean (SEM)	15.38 (4.015)	-2.20 (6.036)	17.57 (7.250)	0.0196
	95% CI for LS MEAN	(7.29, 23.47)	(-14.37, 9.98)	(2.96, 32.19)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning

Subgroup by treatment interaction p-value 0.0390

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning
> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	2.63 (3.323)	0.83 (5.421)	1.79 (6.195)	0.7742
	95% CI for LS MEAN	(-4.16, 9.41)	(-10.22, 11.89)	(-10.88, 14.47)	
Week 4	LS Mean (SEM)	7.14 (2.701)	-1.30 (4.209)	8.44 (4.777)	0.0884
	95% CI for LS MEAN	(1.63, 12.65)	(-9.91, 7.31)	(-1.35, 18.23)	
Week 8	LS Mean (SEM)	1.67 (3.510)	5.71 (5.871)	-4.04 (6.671)	0.5512
	95% CI for LS MEAN	(-5.57, 8.92)	(-6.41, 17.84)	(-17.88, 9.81)	
Week 12	LS Mean (SEM)	5.76 (2.278)	3.33 (3.420)	2.42 (3.850)	0.5342
	95% CI for LS MEAN	(1.10, 10.41)	(-3.67, 10.34)	(-5.47, 10.31)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning
≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	7.16 (1.850)	0.29 (2.729)	6.87 (3.305)	0.0433
	95% CI for LS MEAN	(3.44, 10.89)	(-5.21, 5.79)	(0.22, 13.53)	
Week 4	LS Mean (SEM)	3.13 (3.379)	2.51 (5.015)	0.62 (6.051)	0.9190
	95% CI for LS MEAN	(-3.68, 9.93)	(-7.59, 12.61)	(-11.57, 12.80)	
Week 8	LS Mean (SEM)	9.80 (2.723)	1.22 (4.009)	8.58 (4.844)	0.0855
	95% CI for LS MEAN	(4.27, 15.33)	(-6.92, 9.37)	(-1.26, 18.42)	
Week 12	LS Mean (SEM)	8.81 (2.705)	-3.60 (3.927)	12.41 (4.772)	0.0127
	95% CI for LS MEAN	(3.36, 14.25)	(-11.52, 4.32)	(2.78, 22.03)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning

Subgroup by treatment interaction p-value	0.4194
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning
> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	0.30 (3.702)	-8.28 (5.948)	8.58 (6.643)	0.2062
	95% CI for LS MEAN	(-7.24, 7.84)	(-20.37, 3.81)	(-4.98, 22.13)	
Week 4	LS Mean (SEM)	5.85 (3.277)	-12.63 (5.044)	18.48 (5.600)	0.0025
	95% CI for LS MEAN	(-0.83, 12.54)	(-22.93, -2.33)	(7.04, 29.93)	
Week 8	LS Mean (SEM)	4.83 (4.005)	-7.43 (6.533)	12.26 (7.352)	0.1065
	95% CI for LS MEAN	(-3.34, 13.00)	(-20.76, 5.90)	(-2.80, 27.32)	
Week 12	LS Mean (SEM)	4.34 (3.841)	0.34 (5.877)	4.01 (6.679)	0.5537
	95% CI for LS MEAN	(-3.52, 12.21)	(-11.73, 12.40)	(-9.73, 17.75)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning
≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	9.46 (2.834)	5.86 (4.166)	3.60 (5.086)	0.4824
	95% CI for LS MEAN	(3.75, 15.17)	(-2.54, 14.25)	(-6.64, 13.84)	
Week 4	LS Mean (SEM)	7.51 (3.402)	4.75 (5.047)	2.76 (6.126)	0.6545
	95% CI for LS MEAN	(0.66, 14.35)	(-5.41, 14.90)	(-9.57, 15.08)	
Week 8	LS Mean (SEM)	9.80 (2.685)	6.13 (3.994)	3.67 (4.854)	0.4539
	95% CI for LS MEAN	(4.38, 15.21)	(-1.92, 14.18)	(-6.12, 13.45)	
Week 12	LS Mean (SEM)	9.37 (2.616)	3.64 (3.782)	5.74 (4.646)	0.2233
	95% CI for LS MEAN	(4.11, 14.64)	(-3.99, 11.26)	(-3.62, 15.09)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Modified Randomized Set

Cognitive Functioning

Subgroup by treatment interaction p-value	0.7012
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Modified Randomized Set

Social Functioning
> 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 24)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	7.19 (4.544)	8.81 (7.038)	-1.62 (8.164)	0.8444
	95% CI for LS MEAN	(-2.05, 16.44)	(-5.51, 23.12)	(-18.25, 15.02)	
Week 4	LS Mean (SEM)	9.27 (3.684)	3.21 (5.297)	6.07 (6.172)	0.3336
	95% CI for LS MEAN	(1.75, 16.80)	(-7.62, 14.04)	(-6.55, 18.68)	
Week 8	LS Mean (SEM)	4.67 (4.500)	3.53 (7.053)	1.14 (8.162)	0.8895
	95% CI for LS MEAN	(-4.48, 13.82)	(-10.84, 17.89)	(-15.52, 17.80)	
Week 12	LS Mean (SEM)	5.82 (4.246)	1.35 (6.212)	4.46 (7.292)	0.5455
	95% CI for LS MEAN	(-2.83, 14.46)	(-11.35, 14.06)	(-10.45, 19.37)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning
≤ 2 Transfusions Within 6 Months of Screening

Visit	Statistics	Danicopan (N = 33)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	7.64 (3.117)	-8.15 (4.717)	15.79 (5.653)	0.0083
	95% CI for LS MEAN	(1.31, 13.97)	(-17.72, 1.41)	(4.33, 27.26)	
Week 4	LS Mean (SEM)	7.64 (3.502)	-3.29 (5.323)	10.93 (6.371)	0.0946
	95% CI for LS MEAN	(0.54, 14.74)	(-14.07, 7.49)	(-1.98, 23.83)	
Week 8	LS Mean (SEM)	15.55 (3.659)	-2.91 (5.557)	18.46 (6.652)	0.0111
	95% CI for LS MEAN	(7.93, 23.16)	(-14.43, 8.60)	(4.66, 32.27)	
Week 12	LS Mean (SEM)	13.72 (3.359)	-7.41 (4.866)	21.14 (5.912)	0.0009
	95% CI for LS MEAN	(6.96, 20.49)	(-17.23, 2.40)	(9.22, 33.05)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.10.5

Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning

Subgroup by treatment interaction p-value	0.2259
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional transfusion history variable and transfusion history by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.6
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL
Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.91 (2.379)	2.47 (3.636)	4.44 (4.347)	0.3124
	95% CI for LS MEAN	(2.12, 11.70)	(-4.84, 9.79)	(-4.31, 13.19)	
Week 4	LS Mean (SEM)	9.77 (2.700)	-1.42 (4.126)	11.19 (4.933)	0.0281
	95% CI for LS MEAN	(4.33, 15.20)	(-9.72, 6.89)	(1.26, 21.11)	
Week 8	LS Mean (SEM)	2.95 (3.697)	-1.30 (5.695)	4.25 (6.790)	0.5343
	95% CI for LS MEAN	(-4.49, 10.40)	(-12.76, 10.16)	(-9.41, 17.92)	
Week 12	LS Mean (SEM)	9.12 (2.657)	4.69 (3.982)	4.43 (4.788)	0.3604
	95% CI for LS MEAN	(3.77, 14.48)	(-3.34, 12.73)	(-5.23, 14.09)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.6
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL
Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	4.29 (3.848)	3.06 (6.236)	1.23 (7.134)	0.8638
	95% CI for LS MEAN	(-3.56, 12.14)	(-9.65, 15.76)	(-13.34, 15.81)	
Week 4	LS Mean (SEM)	7.32 (3.361)	0.58 (5.087)	6.74 (5.897)	0.2629
	95% CI for LS MEAN	(0.45, 14.18)	(-9.83, 10.99)	(-5.35, 18.82)	
Week 8	LS Mean (SEM)	17.88 (3.998)	0.46 (6.391)	17.42 (7.344)	0.0270
	95% CI for LS MEAN	(9.60, 26.15)	(-12.75, 13.67)	(2.17, 32.67)	
Week 12	LS Mean (SEM)	14.08 (3.306)	8.91 (4.864)	5.17 (5.680)	0.3705
	95% CI for LS MEAN	(7.33, 20.84)	(-1.05, 18.87)	(-6.47, 16.81)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.6
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL

Subgroup by treatment interaction p-value	0.8687
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.6
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning
Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	7.59 (1.889)	3.15 (2.869)	4.45 (3.445)	0.2034
	95% CI for LS MEAN	(3.79, 11.40)	(-2.63, 8.92)	(-2.49, 11.39)	
Week 4	LS Mean (SEM)	13.31 (2.206)	2.70 (3.379)	10.61 (4.047)	0.0118
	95% CI for LS MEAN	(8.87, 17.75)	(-4.10, 9.51)	(2.46, 18.75)	
Week 8	LS Mean (SEM)	8.73 (2.637)	3.45 (4.055)	5.28 (4.846)	0.2815
	95% CI for LS MEAN	(3.41, 14.05)	(-4.72, 11.62)	(-4.48, 15.05)	
Week 12	LS Mean (SEM)	12.17 (2.453)	0.92 (3.705)	11.24 (4.454)	0.0152
	95% CI for LS MEAN	(7.23, 17.11)	(-6.54, 8.39)	(2.28, 20.21)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.10.6
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning
Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	3.38 (2.225)	-7.26 (3.595)	10.65 (3.990)	0.0126
	95% CI for LS MEAN	(-1.18, 7.94)	(-14.61, 0.08)	(2.47, 18.83)	
Week 4	LS Mean (SEM)	7.63 (2.908)	-6.51 (4.502)	14.14 (5.215)	0.0111
	95% CI for LS MEAN	(1.69, 13.56)	(-15.70, 2.68)	(3.48, 24.80)	
Week 8	LS Mean (SEM)	9.79 (3.097)	-11.01 (4.845)	20.81 (5.620)	0.0013
	95% CI for LS MEAN	(3.38, 16.21)	(-21.04, -0.98)	(9.14, 32.47)	
Week 12	LS Mean (SEM)	8.57 (2.676)	-5.03 (4.065)	13.60 (4.710)	0.0079
	95% CI for LS MEAN	(3.07, 14.06)	(-13.39, 3.33)	(3.90, 23.30)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.6
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning

Subgroup by treatment interaction p-value	0.1363
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.6
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning
Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	7.95 (3.427)	6.72 (5.290)	1.22 (6.302)	0.8471
	95% CI for LS MEAN	(1.04, 14.86)	(-3.95, 17.40)	(-11.49, 13.94)	
Week 4	LS Mean (SEM)	15.88 (3.425)	3.15 (5.414)	12.73 (6.407)	0.0533
	95% CI for LS MEAN	(8.98, 22.78)	(-7.76, 14.07)	(-0.19, 25.64)	
Week 8	LS Mean (SEM)	9.18 (3.589)	3.53 (5.734)	5.65 (6.765)	0.4087
	95% CI for LS MEAN	(1.92, 16.45)	(-8.07, 15.13)	(-8.04, 19.34)	
Week 12	LS Mean (SEM)	13.56 (3.787)	6.72 (5.896)	6.83 (7.008)	0.3346
	95% CI for LS MEAN	(5.94, 21.18)	(-5.15, 18.60)	(-7.28, 20.94)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.6
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning
Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	9.17 (5.691)	-6.31 (9.151)	15.48 (10.300)	0.1466
	95% CI for LS MEAN	(-2.57, 20.91)	(-25.12, 12.51)	(-5.84, 36.80)	
Week 4	LS Mean (SEM)	11.44 (5.747)	-3.34 (8.814)	14.78 (10.085)	0.1538
	95% CI for LS MEAN	(-0.28, 23.17)	(-21.34, 14.67)	(-5.87, 35.43)	
Week 8	LS Mean (SEM)	22.13 (4.677)	-21.37 (7.383)	43.50 (8.100)	<0.0001
	95% CI for LS MEAN	(12.22, 32.03)	(-36.96, -5.78)	(26.25, 60.74)	
Week 12	LS Mean (SEM)	14.07 (5.402)	-3.34 (8.037)	17.41 (9.216)	0.0695
	95% CI for LS MEAN	(3.03, 25.12)	(-19.79, 13.12)	(-1.49, 36.31)	

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Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.6
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning

Subgroup by treatment interaction p-value	0.0631
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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.6
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning
Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.04 (1.924)	-1.35 (2.918)	7.39 (3.501)	0.0402
	95% CI for LS MEAN	(2.16, 9.91)	(-7.23, 4.52)	(0.34, 14.44)	
Week 4	LS Mean (SEM)	6.70 (2.187)	-2.47 (3.345)	9.17 (4.001)	0.0266
	95% CI for LS MEAN	(2.30, 11.11)	(-9.20, 4.27)	(1.11, 17.23)	
Week 8	LS Mean (SEM)	2.85 (2.404)	4.56 (3.717)	-1.71 (4.430)	0.7015
	95% CI for LS MEAN	(-2.03, 7.73)	(-2.98, 12.10)	(-10.70, 7.27)	
Week 12	LS Mean (SEM)	6.66 (2.248)	-3.02 (3.324)	9.68 (4.018)	0.0201
	95% CI for LS MEAN	(2.13, 11.18)	(-9.72, 3.67)	(1.59, 17.77)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.6
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning
Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	3.23 (3.428)	1.42 (5.322)	1.81 (5.959)	0.7641
	95% CI for LS MEAN	(-3.83, 10.28)	(-9.49, 12.33)	(-10.46, 14.08)	
Week 4	LS Mean (SEM)	0.96 (4.841)	5.71 (7.400)	-4.75 (8.605)	0.5854
	95% CI for LS MEAN	(-8.94, 10.85)	(-9.45, 20.86)	(-22.41, 12.91)	
Week 8	LS Mean (SEM)	10.17 (4.472)	-2.05 (6.904)	12.23 (7.952)	0.1408
	95% CI for LS MEAN	(0.85, 19.50)	(-16.43, 12.32)	(-4.43, 28.89)	
Week 12	LS Mean (SEM)	8.28 (3.489)	1.08 (5.153)	7.20 (5.886)	0.2317
	95% CI for LS MEAN	(1.14, 15.42)	(-9.48, 11.64)	(-4.87, 19.27)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:47:53

Data cutoff date of 31Mar2023
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Table GERMANY.2.5.10.6
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning

Subgroup by treatment interaction p-value	0.6086
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.6
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning
Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.04 (2.966)	-0.55 (4.515)	6.58 (5.435)	0.2317
	95% CI for LS MEAN	(0.07, 12.00)	(-9.63, 8.54)	(-4.35, 17.51)	
Week 4	LS Mean (SEM)	8.59 (2.822)	-1.66 (4.326)	10.24 (5.199)	0.0547
	95% CI for LS MEAN	(2.91, 14.26)	(-10.36, 7.05)	(-0.22, 20.70)	
Week 8	LS Mean (SEM)	7.52 (2.631)	7.02 (4.077)	0.50 (4.885)	0.9196
	95% CI for LS MEAN	(2.21, 12.82)	(-1.19, 15.23)	(-9.34, 10.34)	
Week 12	LS Mean (SEM)	6.60 (2.761)	7.23 (4.122)	-0.63 (4.994)	0.9003
	95% CI for LS MEAN	(1.04, 12.17)	(-1.08, 15.54)	(-10.69, 9.44)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:47:53

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Table GERMANY.2.5.10.6
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning
Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	3.94 (3.247)	1.60 (5.278)	2.34 (5.870)	0.6932
	95% CI for LS MEAN	(-2.71, 10.58)	(-9.18, 12.37)	(-9.69, 14.37)	
Week 4	LS Mean (SEM)	3.18 (4.392)	-3.69 (6.763)	6.87 (7.859)	0.3894
	95% CI for LS MEAN	(-5.78, 12.13)	(-17.50, 10.12)	(-9.21, 22.94)	
Week 8	LS Mean (SEM)	7.20 (3.871)	-8.62 (6.160)	15.82 (7.032)	0.0339
	95% CI for LS MEAN	(-0.77, 15.17)	(-21.27, 4.03)	(1.31, 30.33)	
Week 12	LS Mean (SEM)	7.78 (3.254)	-7.39 (4.853)	15.17 (5.575)	0.0114
	95% CI for LS MEAN	(1.12, 14.44)	(-17.35, 2.57)	(3.71, 26.63)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:47:53

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Table GERMANY.2.5.10.6
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning

Subgroup by treatment interaction p-value	0.1679
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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.6
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning
Screening Hgb Level < 8.5 g/dL

Visit	Statistics	Danicopan (N = 35)	Placebo (N = 15)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	8.85 (3.289)	3.78 (5.125)	5.07 (6.093)	0.4103
	95% CI for LS MEAN	(2.21, 15.49)	(-6.55, 14.12)	(-7.23, 17.36)	
Week 4	LS Mean (SEM)	12.19 (2.925)	1.67 (4.557)	10.51 (5.419)	0.0592
	95% CI for LS MEAN	(6.28, 18.09)	(-7.52, 10.87)	(-0.43, 21.45)	
Week 8	LS Mean (SEM)	9.11 (3.244)	5.18 (5.082)	3.93 (6.035)	0.5194
	95% CI for LS MEAN	(2.51, 15.71)	(-5.13, 15.50)	(-8.33, 16.19)	
Week 12	LS Mean (SEM)	9.38 (3.663)	-2.60 (5.517)	11.98 (6.626)	0.0776
	95% CI for LS MEAN	(2.00, 16.76)	(-13.72, 8.53)	(-1.38, 25.33)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:47:53

Data cutoff date of 31Mar2023
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Table GERMANY.2.5.10.6
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning
Screening Hgb Level ≥ 8.5 g/dL

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 9)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.20 (3.464)	-13.47 (5.321)	19.67 (5.953)	0.0028
	95% CI for LS MEAN	(-0.92, 13.31)	(-24.37, -2.57)	(7.42, 31.93)	
Week 4	LS Mean (SEM)	3.17 (4.377)	-6.22 (6.588)	9.39 (7.624)	0.2281
	95% CI for LS MEAN	(-5.76, 12.10)	(-19.69, 7.24)	(-6.22, 25.00)	
Week 8	LS Mean (SEM)	15.41 (5.464)	-9.10 (8.845)	24.52 (10.110)	0.0285
	95% CI for LS MEAN	(3.83, 27.00)	(-27.83, 9.62)	(2.96, 46.08)	
Week 12	LS Mean (SEM)	11.80 (3.748)	-8.08 (5.394)	19.87 (6.232)	0.0036
	95% CI for LS MEAN	(4.13, 19.46)	(-19.14, 2.99)	(7.08, 32.67)	

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Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.10.6
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning

Subgroup by treatment interaction p-value	0.1592
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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional screening Hgb level variable and screening Hgb level by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.7
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL
Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.76 (3.308)	2.55 (4.993)	3.21 (5.768)	0.5822
	95% CI for LS MEAN	(-1.03, 12.55)	(-7.66, 12.75)	(-8.62, 15.04)	
Week 4	LS Mean (SEM)	3.78 (3.209)	-1.97 (4.690)	5.75 (5.481)	0.3039
	95% CI for LS MEAN	(-2.80, 10.36)	(-11.59, 7.65)	(-5.50, 16.99)	
Week 8	LS Mean (SEM)	6.45 (5.347)	-4.69 (8.455)	11.15 (9.871)	0.2697
	95% CI for LS MEAN	(-4.60, 17.50)	(-22.06, 12.68)	(-9.20, 31.50)	
Week 12	LS Mean (SEM)	8.51 (4.018)	-1.97 (5.625)	10.48 (6.746)	0.1367
	95% CI for LS MEAN	(0.16, 16.87)	(-13.73, 9.79)	(-3.64, 24.60)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.7
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL
Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.64 (2.800)	2.59 (4.433)	4.05 (5.192)	0.4391
	95% CI for LS MEAN	(1.01, 12.27)	(-6.33, 11.51)	(-6.40, 14.50)	
Week 4	LS Mean (SEM)	12.43 (2.746)	-0.39 (4.345)	12.82 (5.087)	0.0153
	95% CI for LS MEAN	(6.90, 17.95)	(-9.13, 8.36)	(2.58, 23.05)	
Week 8	LS Mean (SEM)	10.28 (3.521)	2.07 (5.463)	8.21 (6.456)	0.2103
	95% CI for LS MEAN	(3.19, 17.37)	(-8.95, 13.08)	(-4.81, 21.24)	
Week 12	LS Mean (SEM)	12.74 (2.438)	11.52 (3.818)	1.22 (4.469)	0.7863
	95% CI for LS MEAN	(7.83, 17.65)	(3.82, 19.21)	(-7.79, 10.23)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:47:55

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Table GERMANY.2.5.10.7
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL

Subgroup by treatment interaction p-value	0.9796
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.7
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning
Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	4.02 (2.274)	0.76 (3.413)	3.25 (3.960)	0.4190
	95% CI for LS MEAN	(-0.66, 8.70)	(-6.23, 7.76)	(-4.89, 11.40)	
Week 4	LS Mean (SEM)	8.78 (2.446)	-7.52 (3.573)	16.30 (4.207)	0.0006
	95% CI for LS MEAN	(3.78, 13.78)	(-14.83, -0.21)	(7.68, 24.92)	
Week 8	LS Mean (SEM)	6.82 (3.379)	-13.66 (5.016)	20.48 (5.993)	0.0022
	95% CI for LS MEAN	(-0.15, 13.80)	(-23.96, -3.35)	(8.12, 32.83)	
Week 12	LS Mean (SEM)	8.12 (2.955)	-7.52 (4.173)	15.65 (5.018)	0.0042
	95% CI for LS MEAN	(2.09, 14.16)	(-16.07, 1.02)	(5.36, 25.93)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.7
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning
Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	7.59 (2.009)	-2.99 (3.134)	10.59 (3.717)	0.0066
	95% CI for LS MEAN	(3.55, 11.64)	(-9.31, 3.32)	(3.10, 18.07)	
Week 4	LS Mean (SEM)	12.95 (2.286)	2.24 (3.601)	10.71 (4.259)	0.0155
	95% CI for LS MEAN	(8.35, 17.55)	(-5.00, 9.49)	(2.14, 19.28)	
Week 8	LS Mean (SEM)	10.82 (2.359)	3.30 (3.669)	7.53 (4.356)	0.0913
	95% CI for LS MEAN	(6.07, 15.57)	(-4.11, 10.70)	(-1.26, 16.32)	
Week 12	LS Mean (SEM)	13.35 (2.032)	1.29 (3.172)	12.06 (3.761)	0.0025
	95% CI for LS MEAN	(9.26, 17.45)	(-5.11, 7.69)	(4.48, 19.64)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.10.7
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning

Subgroup by treatment interaction p-value	0.7882
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.7
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning
Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-0.15 (4.906)	7.48 (8.100)	-7.62 (9.221)	0.4162
	95% CI for LS MEAN	(-10.26, 9.97)	(-9.15, 24.10)	(-26.61, 11.36)	
Week 4	LS Mean (SEM)	2.24 (5.477)	0.56 (8.486)	1.67 (9.979)	0.8681
	95% CI for LS MEAN	(-9.01, 13.48)	(-16.84, 17.97)	(-18.82, 22.16)	
Week 8	LS Mean (SEM)	3.31 (5.595)	-17.44 (8.875)	20.75 (10.359)	0.0572
	95% CI for LS MEAN	(-8.30, 14.92)	(-35.74, 0.87)	(-0.69, 42.18)	
Week 12	LS Mean (SEM)	3.42 (6.331)	4.27 (9.620)	-0.85 (11.408)	0.9415
	95% CI for LS MEAN	(-9.56, 16.40)	(-15.47, 24.00)	(-24.26, 22.57)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:47:55

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.7
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning
Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	12.04 (3.559)	0.09 (5.522)	11.95 (6.514)	0.0735
	95% CI for LS MEAN	(4.87, 19.22)	(-11.05, 11.23)	(-1.19, 25.10)	
Week 4	LS Mean (SEM)	19.63 (3.416)	2.47 (5.404)	17.16 (6.324)	0.0094
	95% CI for LS MEAN	(12.76, 26.50)	(-8.41, 13.35)	(4.43, 29.89)	
Week 8	LS Mean (SEM)	17.96 (3.710)	-3.13 (5.715)	21.09 (6.741)	0.0033
	95% CI for LS MEAN	(10.48, 25.43)	(-14.68, 8.41)	(7.47, 34.71)	
Week 12	LS Mean (SEM)	18.36 (3.253)	3.66 (5.104)	14.70 (5.977)	0.0179
	95% CI for LS MEAN	(11.81, 24.90)	(-6.62, 13.94)	(2.66, 26.74)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:47:55

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.7
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning

Subgroup by treatment interaction p-value 0.1719

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.7
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning
Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	4.41 (2.993)	-1.66 (4.509)	6.07 (5.196)	0.2522
	95% CI for LS MEAN	(-1.72, 10.55)	(-10.87, 7.55)	(-4.57, 16.72)	
Week 4	LS Mean (SEM)	0.45 (4.147)	-1.14 (6.043)	1.59 (7.191)	0.8271
	95% CI for LS MEAN	(-8.04, 8.93)	(-13.50, 11.22)	(-13.15, 16.32)	
Week 8	LS Mean (SEM)	5.16 (2.641)	12.40 (4.282)	-7.24 (4.778)	0.1411
	95% CI for LS MEAN	(-0.27, 10.59)	(3.64, 21.16)	(-17.04, 2.56)	
Week 12	LS Mean (SEM)	1.21 (4.110)	-3.64 (5.747)	4.85 (6.902)	0.4887
	95% CI for LS MEAN	(-7.22, 9.65)	(-15.47, 8.19)	(-9.37, 19.08)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.7
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning
Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.47 (2.128)	0.27 (3.346)	5.20 (3.921)	0.1915
	95% CI for LS MEAN	(1.18, 9.75)	(-6.47, 7.01)	(-2.70, 13.10)	
Week 4	LS Mean (SEM)	7.13 (2.664)	1.46 (4.241)	5.67 (4.971)	0.2599
	95% CI for LS MEAN	(1.77, 12.48)	(-7.07, 9.99)	(-4.33, 15.67)	
Week 8	LS Mean (SEM)	6.34 (2.675)	-0.24 (4.133)	6.58 (4.876)	0.1844
	95% CI for LS MEAN	(0.95, 11.73)	(-8.59, 8.10)	(-3.27, 16.43)	
Week 12	LS Mean (SEM)	9.24 (2.039)	-0.33 (3.204)	9.57 (3.749)	0.0142
	95% CI for LS MEAN	(5.14, 13.34)	(-6.78, 6.13)	(2.02, 17.12)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.7
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning

Subgroup by treatment interaction p-value	0.6034
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.7
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning
Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	4.19 (4.239)	-7.81 (6.206)	12.00 (7.416)	0.1174
	95% CI for LS MEAN	(-4.52, 12.90)	(-20.53, 4.90)	(-3.23, 27.23)	
Week 4	LS Mean (SEM)	2.60 (4.070)	-11.60 (5.880)	14.20 (7.040)	0.0536
	95% CI for LS MEAN	(-5.73, 10.93)	(-23.64, 0.44)	(-0.24, 28.63)	
Week 8	LS Mean (SEM)	7.11 (3.113)	9.55 (5.117)	-2.44 (5.855)	0.6804
	95% CI for LS MEAN	(0.68, 13.54)	(-0.91, 20.01)	(-14.45, 9.58)	
Week 12	LS Mean (SEM)	3.51 (3.644)	0.07 (4.931)	3.44 (5.999)	0.5708
	95% CI for LS MEAN	(-3.95, 10.98)	(-10.05, 10.19)	(-8.88, 15.76)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.7
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning
Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.24 (2.522)	4.50 (3.967)	0.74 (4.650)	0.8740
	95% CI for LS MEAN	(0.16, 10.31)	(-3.49, 12.48)	(-8.62, 10.10)	
Week 4	LS Mean (SEM)	8.11 (2.880)	3.31 (4.574)	4.81 (5.358)	0.3743
	95% CI for LS MEAN	(2.32, 13.90)	(-5.89, 12.50)	(-5.97, 15.58)	
Week 8	LS Mean (SEM)	7.32 (2.873)	0.73 (4.485)	6.59 (5.280)	0.2189
	95% CI for LS MEAN	(1.54, 13.10)	(-8.31, 9.77)	(-4.06, 17.23)	
Week 12	LS Mean (SEM)	8.31 (2.500)	2.11 (3.931)	6.19 (4.603)	0.1856
	95% CI for LS MEAN	(3.27, 13.34)	(-5.81, 10.04)	(-3.09, 15.47)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
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Table GERMANY.2.5.10.7
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning

Subgroup by treatment interaction p-value	0.4632
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.7
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning
Background C5 Inhibitor Therapy Eculizumab

Visit	Statistics	Danicopan (N = 21)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	0.52 (4.427)	-3.90 (6.808)	4.42 (7.920)	0.5817
	95% CI for LS MEAN	(-8.64, 9.67)	(-17.87, 10.06)	(-11.88, 20.72)	
Week 4	LS Mean (SEM)	2.90 (4.204)	-14.38 (6.230)	17.27 (7.333)	0.0286
	95% CI for LS MEAN	(-5.87, 11.66)	(-27.34, -1.41)	(1.99, 32.55)	
Week 8	LS Mean (SEM)	6.65 (5.861)	21.01 (10.395)	-14.36 (11.733)	0.2439
	95% CI for LS MEAN	(-6.29, 19.59)	(-1.37, 43.39)	(-39.86, 11.14)	
Week 12	LS Mean (SEM)	3.45 (5.738)	-15.64 (7.908)	19.10 (9.559)	0.0571
	95% CI for LS MEAN	(-8.33, 15.23)	(-31.96, 0.67)	(-0.63, 38.82)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.7
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning
Background C5 Inhibitor Therapy Ravulizumab

Visit	Statistics	Danicopan (N = 36)	Placebo (N = 14)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	11.30 (2.972)	-2.08 (4.705)	13.38 (5.503)	0.0190
	95% CI for LS MEAN	(5.32, 17.28)	(-11.55, 7.39)	(2.30, 24.46)	
Week 4	LS Mean (SEM)	11.30 (2.856)	6.25 (4.515)	5.05 (5.278)	0.3439
	95% CI for LS MEAN	(5.55, 17.04)	(-2.84, 15.34)	(-5.58, 15.67)	
Week 8	LS Mean (SEM)	14.12 (2.972)	-1.62 (4.586)	15.74 (5.393)	0.0059
	95% CI for LS MEAN	(8.12, 20.12)	(-10.90, 7.67)	(4.82, 26.66)	
Week 12	LS Mean (SEM)	13.68 (2.514)	1.49 (3.930)	12.19 (4.590)	0.0110
	95% CI for LS MEAN	(8.61, 18.74)	(-6.43, 9.41)	(2.94, 21.44)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional background C5 inhibitor therapy variable and background C5 inhibitor therapy by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:47:55

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.7
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning

Subgroup by treatment interaction p-value	0.7021
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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.8
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL
Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	51.00 (21.769)	-15.67 (22.510)	66.67 (32.724)	0.0905
	95% CI for LS MEAN	(-1.07, 103.07)	(-74.22, 42.89)	(-14.54, 147.88)	
Week 4	LS Mean (SEM)	57.25 (19.878)	-21.92 (18.685)	79.17 (28.888)	0.0405
	95% CI for LS MEAN	(8.96, 105.55)	(-73.87, 30.04)	(5.05, 153.29)	
Week 8	LS Mean (SEM)	51.00 (21.876)	-15.67 (22.716)	66.67 (32.937)	0.0908
	95% CI for LS MEAN	(-1.05, 103.05)	(-74.20, 42.87)	(-14.50, 147.84)	
Week 12	LS Mean (SEM)	56.21 (21.872)	-11.50 (22.708)	67.71 (32.928)	0.0849
	95% CI for LS MEAN	(4.61, 107.81)	(-69.28, 46.28)	(-12.60, 148.02)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.8
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL
Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.12 (2.252)	1.24 (3.547)	4.88 (4.128)	0.2413
	95% CI for LS MEAN	(1.63, 10.62)	(-5.83, 8.32)	(-3.36, 13.12)	
Week 4	LS Mean (SEM)	8.51 (2.304)	-1.52 (3.555)	10.03 (4.170)	0.0190
	95% CI for LS MEAN	(3.91, 13.10)	(-8.62, 5.58)	(1.70, 18.35)	
Week 8	LS Mean (SEM)	8.71 (3.309)	-4.72 (5.262)	13.42 (6.171)	0.0337
	95% CI for LS MEAN	(2.09, 15.33)	(-15.24, 5.81)	(1.07, 25.78)	
Week 12	LS Mean (SEM)	11.24 (2.153)	4.73 (3.230)	6.51 (3.809)	0.0930
	95% CI for LS MEAN	(6.93, 15.55)	(-1.74, 11.20)	(-1.12, 14.14)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.8

Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL

Subgroup by treatment interaction p-value	0.6538
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

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Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.8
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning
Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	7.24 (11.333)	-3.32 (6.195)	10.56 (14.707)	0.4938
	95% CI for LS MEAN	(-19.14, 33.61)	(-17.98, 11.34)	(-23.49, 44.60)	
Week 4	LS Mean (SEM)	14.74 (11.572)	-1.65 (7.024)	16.39 (15.256)	0.3106
	95% CI for LS MEAN	(-11.68, 41.15)	(-17.95, 14.64)	(-18.11, 50.89)	
Week 8	LS Mean (SEM)	9.74 (11.349)	0.01 (6.252)	9.72 (14.743)	0.5281
	95% CI for LS MEAN	(-16.61, 36.09)	(-14.52, 14.55)	(-24.26, 43.71)	
Week 12	LS Mean (SEM)	14.74 (11.474)	-4.99 (6.694)	19.72 (15.032)	0.2235
	95% CI for LS MEAN	(-11.64, 41.11)	(-20.55, 10.58)	(-14.53, 53.98)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:47:57

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.8
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning
Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.47 (1.680)	-1.49 (2.625)	7.96 (3.061)	0.0116
	95% CI for LS MEAN	(3.11, 9.82)	(-6.73, 3.75)	(1.84, 14.07)	
Week 4	LS Mean (SEM)	11.17 (2.024)	-2.00 (3.128)	13.17 (3.687)	0.0007
	95% CI for LS MEAN	(7.13, 15.21)	(-8.25, 4.25)	(5.81, 20.53)	
Week 8	LS Mean (SEM)	9.67 (2.320)	-3.55 (3.632)	13.22 (4.278)	0.0030
	95% CI for LS MEAN	(5.03, 14.30)	(-10.81, 3.70)	(4.67, 21.77)	
Week 12	LS Mean (SEM)	10.69 (2.070)	-2.00 (3.140)	12.69 (3.723)	0.0012
	95% CI for LS MEAN	(6.56, 14.82)	(-8.28, 4.28)	(5.25, 20.13)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:47:57

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Table GERMANY.2.5.10.8

Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning

Subgroup by treatment interaction p-value	0.6088
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

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Table GERMANY.2.5.10.8
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning
Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-33.83 (14.392)	22.75 (13.864)	-56.58 (22.589)	0.0269
	95% CI for LS MEAN	(-64.78, -2.88)	(-8.65, 54.16)	(-105.57, -7.60)	
Week 4	LS Mean (SEM)	-19.25 (11.600)	22.75 (6.864)	-42.00 (17.106)	0.0358
	95% CI for LS MEAN	(-45.58, 7.08)	(6.96, 38.54)	(-80.54, -3.46)	
Week 8	LS Mean (SEM)	-21.33 (12.372)	10.25 (9.172)	-31.58 (18.659)	0.1131
	95% CI for LS MEAN	(-48.05, 5.39)	(-10.06, 30.57)	(-71.67, 8.51)	
Week 12	LS Mean (SEM)	-13.00 (11.970)	10.25 (8.033)	-23.25 (17.853)	0.2189
	95% CI for LS MEAN	(-39.42, 13.43)	(-8.17, 28.67)	(-62.44, 15.94)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.8
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning
Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	9.11 (3.195)	1.22 (5.118)	7.89 (5.926)	0.1880
	95% CI for LS MEAN	(2.72, 15.50)	(-9.01, 11.45)	(-3.96, 19.74)	
Week 4	LS Mean (SEM)	13.32 (3.468)	0.12 (5.485)	13.21 (6.401)	0.0432
	95% CI for LS MEAN	(6.40, 20.25)	(-10.84, 11.07)	(0.42, 25.99)	
Week 8	LS Mean (SEM)	13.39 (3.780)	-6.83 (6.139)	20.22 (7.114)	0.0065
	95% CI for LS MEAN	(5.79, 20.98)	(-19.16, 5.49)	(5.92, 34.52)	
Week 12	LS Mean (SEM)	11.67 (3.554)	5.38 (5.511)	6.29 (6.469)	0.3346
	95% CI for LS MEAN	(4.58, 18.76)	(-5.63, 16.39)	(-6.64, 19.22)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.8

Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning

Subgroup by treatment interaction p-value	0.9111
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.8
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning
Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.12 (7.137)	-7.38 (6.328)	12.49 (10.315)	0.2488
	95% CI for LS MEAN	(-10.45, 20.68)	(-21.52, 6.77)	(-9.95, 34.93)	
Week 4	LS Mean (SEM)	8.24 (6.322)	-7.38 (4.254)	15.62 (8.572)	0.1052
	95% CI for LS MEAN	(-6.39, 22.87)	(-17.15, 2.40)	(-4.08, 35.32)	
Week 8	LS Mean (SEM)	5.12 (6.974)	-9.46 (5.952)	14.58 (9.974)	0.1693
	95% CI for LS MEAN	(-10.16, 20.39)	(-22.66, 3.74)	(-7.13, 36.28)	
Week 12	LS Mean (SEM)	9.28 (6.882)	-11.54 (5.734)	20.83 (9.780)	0.0548
	95% CI for LS MEAN	(-5.85, 24.42)	(-24.24, 1.15)	(-0.51, 42.16)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.8
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning
Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.37 (1.906)	0.72 (2.997)	5.65 (3.484)	0.1098
	95% CI for LS MEAN	(2.56, 10.18)	(-5.26, 6.71)	(-1.31, 12.61)	
Week 4	LS Mean (SEM)	5.34 (2.604)	1.91 (4.053)	3.43 (4.772)	0.4751
	95% CI for LS MEAN	(0.14, 10.53)	(-6.18, 10.00)	(-6.10, 12.95)	
Week 8	LS Mean (SEM)	7.63 (2.352)	5.33 (3.744)	2.30 (4.353)	0.5993
	95% CI for LS MEAN	(2.92, 12.34)	(-2.16, 12.83)	(-6.42, 11.03)	
Week 12	LS Mean (SEM)	7.99 (2.136)	0.24 (3.227)	7.74 (3.812)	0.0465
	95% CI for LS MEAN	(3.72, 12.25)	(-6.21, 6.70)	(0.13, 15.36)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.8

Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning

Subgroup by treatment interaction p-value	0.7150
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Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning
Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	24.02 (9.135)	-14.71 (7.189)	38.73 (13.347)	0.0170
	95% CI for LS MEAN	(3.55, 44.49)	(-32.17, 2.76)	(8.68, 68.77)	
Week 4	LS Mean (SEM)	36.52 (9.056)	-14.71 (6.986)	51.23 (13.184)	0.0024
	95% CI for LS MEAN	(16.50, 56.54)	(-30.25, 0.83)	(22.30, 80.15)	
Week 8	LS Mean (SEM)	34.44 (9.437)	-14.71 (7.931)	49.14 (13.963)	0.0043
	95% CI for LS MEAN	(13.80, 55.07)	(-32.64, 3.23)	(18.66, 79.63)	
Week 12	LS Mean (SEM)	30.27 (9.675)	-14.71 (8.486)	44.98 (14.444)	0.0088
	95% CI for LS MEAN	(9.24, 51.30)	(-33.85, 4.44)	(13.56, 76.39)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.8
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning
Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.70 (2.523)	0.63 (3.968)	6.07 (4.651)	0.1965
	95% CI for LS MEAN	(1.66, 11.74)	(-7.28, 8.55)	(-3.22, 15.35)	
Week 4	LS Mean (SEM)	6.02 (2.690)	-2.32 (4.177)	8.35 (4.923)	0.0946
	95% CI for LS MEAN	(0.66, 11.39)	(-10.66, 6.01)	(-1.48, 18.17)	
Week 8	LS Mean (SEM)	7.31 (2.487)	1.93 (3.944)	5.38 (4.604)	0.2472
	95% CI for LS MEAN	(2.34, 12.28)	(-5.94, 9.81)	(-3.82, 14.58)	
Week 12	LS Mean (SEM)	7.72 (2.301)	2.68 (3.471)	5.05 (4.107)	0.2238
	95% CI for LS MEAN	(3.13, 12.32)	(-4.26, 9.62)	(-3.16, 13.26)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:47:57

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Table GERMANY.2.5.10.8

Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning

Subgroup by treatment interaction p-value	0.8684
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.8
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning
Patients Enrolled from Japan Yes

Visit	Statistics	Danicopan (N = 8)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	-1.32 (20.656)	7.82 (13.239)	-9.13 (30.406)	0.7723
	95% CI for LS MEAN	(-49.21, 46.58)	(-30.82, 46.45)	(-80.58, 62.31)	
Week 4	LS Mean (SEM)	2.85 (20.200)	11.98 (11.747)	-9.13 (29.472)	0.7648
	95% CI for LS MEAN	(-44.12, 49.82)	(-15.45, 39.42)	(-77.43, 59.16)	
Week 8	LS Mean (SEM)	-1.32 (20.232)	7.82 (11.857)	-9.13 (29.538)	0.7653
	95% CI for LS MEAN	(-48.32, 45.69)	(-20.55, 36.19)	(-77.60, 59.33)	
Week 12	LS Mean (SEM)	2.85 (20.216)	7.82 (11.801)	-4.97 (29.505)	0.8708
	95% CI for LS MEAN	(-44.34, 50.04)	(-21.83, 37.46)	(-73.88, 63.94)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.8
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning
Patients Enrolled from Japan No

Visit	Statistics	Danicopan (N = 49)	Placebo (N = 20)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	7.81 (2.800)	-4.60 (4.445)	12.41 (5.184)	0.0197
	95% CI for LS MEAN	(2.21, 13.41)	(-13.48, 4.28)	(2.05, 22.77)	
Week 4	LS Mean (SEM)	8.15 (2.794)	-3.81 (4.379)	11.96 (5.130)	0.0231
	95% CI for LS MEAN	(2.56, 13.74)	(-12.57, 4.95)	(1.70, 22.23)	
Week 8	LS Mean (SEM)	11.66 (3.216)	-0.53 (5.213)	12.18 (6.048)	0.0499
	95% CI for LS MEAN	(5.18, 18.13)	(-11.01, 9.95)	(0.01, 24.36)	
Week 12	LS Mean (SEM)	10.28 (3.008)	-7.07 (4.532)	17.36 (5.365)	0.0020
	95% CI for LS MEAN	(4.28, 16.29)	(-16.14, 1.99)	(6.63, 28.09)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.8

Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning

Subgroup by treatment interaction p-value	0.4271
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional Japanese patients variable and Japanese patients by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL
Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	7.45 (3.304)	12.20 (4.685)	-4.75 (5.363)	0.3834
	95% CI for LS MEAN	(0.70, 14.21)	(2.62, 21.79)	(-15.74, 6.24)	
Week 4	LS Mean (SEM)	9.73 (3.877)	5.54 (5.567)	4.19 (6.474)	0.5227
	95% CI for LS MEAN	(1.82, 17.63)	(-5.83, 16.91)	(-9.06, 17.44)	
Week 8	LS Mean (SEM)	11.28 (4.026)	8.87 (5.535)	2.41 (6.539)	0.7152
	95% CI for LS MEAN	(3.07, 19.49)	(-2.45, 20.19)	(-10.98, 15.80)	
Week 12	LS Mean (SEM)	13.43 (3.642)	16.37 (5.091)	-2.94 (5.926)	0.6230
	95% CI for LS MEAN	(6.00, 20.85)	(5.96, 26.78)	(-15.07, 9.18)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:47:59

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL
White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.07 (2.800)	-6.82 (4.855)	12.89 (5.582)	0.0268
	95% CI for LS MEAN	(0.38, 11.76)	(-16.66, 3.02)	(1.57, 24.21)	
Week 4	LS Mean (SEM)	8.45 (2.822)	-6.12 (4.743)	14.57 (5.501)	0.0122
	95% CI for LS MEAN	(2.72, 14.19)	(-15.76, 3.52)	(3.39, 25.76)	
Week 8	LS Mean (SEM)	6.27 (4.181)	-4.08 (7.342)	10.35 (8.434)	0.2286
	95% CI for LS MEAN	(-2.25, 14.79)	(-19.02, 10.85)	(-6.82, 27.52)	
Week 12	LS Mean (SEM)	8.69 (2.856)	-0.29 (4.644)	8.98 (5.439)	0.1104
	95% CI for LS MEAN	(2.84, 14.54)	(-9.83, 9.25)	(-2.18, 20.14)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL
Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.96 (7.446)	2.47 (9.403)	4.49 (11.586)	0.7016
	95% CI for LS MEAN	(-8.34, 22.25)	(-16.82, 21.76)	(-19.28, 28.26)	
Week 4	LS Mean (SEM)	14.10 (7.446)	-1.70 (9.403)	15.80 (11.586)	0.1840
	95% CI for LS MEAN	(-1.20, 29.40)	(-20.99, 17.60)	(-7.97, 39.57)	
Week 8	LS Mean (SEM)	12.56 (9.429)	-26.63 (21.461)	39.19 (22.901)	0.1006
	95% CI for LS MEAN	(-6.83, 31.95)	(-71.43, 18.16)	(-8.20, 86.59)	
Week 12	LS Mean (SEM)	20.05 (7.446)	-1.70 (9.403)	21.75 (11.586)	0.0713
	95% CI for LS MEAN	(4.76, 35.35)	(-20.99, 17.60)	(-2.02, 45.52)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Global Health Status/QoL

Subgroup by treatment interaction p-value	0.0922
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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning
Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.33 (2.593)	2.04 (3.657)	4.30 (4.277)	0.3238
	95% CI for LS MEAN	(1.03, 11.63)	(-5.45, 9.52)	(-4.47, 13.06)	
Week 4	LS Mean (SEM)	9.06 (2.560)	6.70 (3.606)	2.36 (4.214)	0.5803
	95% CI for LS MEAN	(3.83, 14.29)	(-0.67, 14.07)	(-6.27, 10.98)	
Week 8	LS Mean (SEM)	7.69 (2.527)	8.04 (3.468)	-0.35 (4.080)	0.9330
	95% CI for LS MEAN	(2.51, 12.87)	(0.91, 15.16)	(-8.73, 8.04)	
Week 12	LS Mean (SEM)	10.58 (2.711)	4.04 (3.805)	6.54 (4.473)	0.1544
	95% CI for LS MEAN	(5.05, 16.11)	(-3.75, 11.82)	(-2.61, 15.70)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning
White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	4.55 (1.670)	-4.47 (2.875)	9.02 (3.306)	0.0101
	95% CI for LS MEAN	(1.15, 7.95)	(-10.31, 1.38)	(2.30, 15.74)	
Week 4	LS Mean (SEM)	10.98 (2.511)	-5.24 (4.199)	16.22 (4.884)	0.0022
	95% CI for LS MEAN	(5.87, 16.09)	(-13.79, 3.30)	(6.28, 26.16)	
Week 8	LS Mean (SEM)	8.35 (3.061)	-7.78 (5.205)	16.13 (6.032)	0.0117
	95% CI for LS MEAN	(2.11, 14.58)	(-18.38, 2.81)	(3.85, 28.41)	
Week 12	LS Mean (SEM)	9.87 (2.537)	-2.58 (4.146)	12.45 (4.853)	0.0156
	95% CI for LS MEAN	(4.70, 15.05)	(-11.05, 5.90)	(2.53, 22.37)	

EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning
Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	15.21 (5.637)	-5.48 (7.064)	20.69 (7.931)	0.0254
	95% CI for LS MEAN	(2.39, 28.03)	(-21.45, 10.49)	(3.10, 38.28)	
Week 4	LS Mean (SEM)	21.88 (5.637)	-15.48 (7.064)	37.36 (7.931)	0.0008
	95% CI for LS MEAN	(9.06, 34.69)	(-31.45, 0.49)	(19.77, 54.94)	
Week 8	LS Mean (SEM)	18.44 (6.469)	-34.91 (13.000)	53.35 (13.794)	0.0011
	95% CI for LS MEAN	(4.32, 32.55)	(-62.35, -7.47)	(24.37, 82.32)	
Week 12	LS Mean (SEM)	18.07 (5.637)	-18.81 (7.064)	36.88 (7.931)	0.0008
	95% CI for LS MEAN	(5.25, 30.88)	(-34.78, -2.84)	(19.29, 54.47)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.9

Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Physical Functioning

Subgroup by treatment interaction p-value	0.0051
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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning
Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	8.38 (4.567)	4.87 (6.542)	3.51 (7.647)	0.6502
	95% CI for LS MEAN	(-0.97, 17.73)	(-8.56, 18.30)	(-12.23, 19.25)	
Week 4	LS Mean (SEM)	13.69 (3.633)	1.54 (5.093)	12.15 (5.828)	0.0469
	95% CI for LS MEAN	(6.25, 21.12)	(-8.90, 11.98)	(0.18, 24.11)	
Week 8	LS Mean (SEM)	11.85 (4.527)	-0.13 (6.133)	11.97 (7.281)	0.1134
	95% CI for LS MEAN	(2.56, 21.13)	(-12.78, 12.53)	(-3.07, 27.02)	
Week 12	LS Mean (SEM)	14.20 (4.362)	3.21 (6.097)	10.99 (7.144)	0.1379
	95% CI for LS MEAN	(5.24, 23.15)	(-9.39, 15.80)	(-3.80, 25.78)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning
White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	8.82 (4.343)	-1.78 (7.471)	10.60 (8.625)	0.2285
	95% CI for LS MEAN	(-0.05, 17.68)	(-17.01, 13.45)	(-6.99, 28.18)	
Week 4	LS Mean (SEM)	16.36 (4.601)	6.22 (7.725)	10.15 (8.984)	0.2668
	95% CI for LS MEAN	(7.01, 25.72)	(-9.49, 21.92)	(-8.12, 28.41)	
Week 8	LS Mean (SEM)	13.34 (4.563)	-7.57 (7.869)	20.90 (9.078)	0.0291
	95% CI for LS MEAN	(3.96, 22.71)	(-23.68, 8.54)	(2.30, 39.51)	
Week 12	LS Mean (SEM)	13.78 (4.499)	14.55 (7.371)	-0.77 (8.627)	0.9295
	95% CI for LS MEAN	(4.63, 22.94)	(-0.48, 29.58)	(-18.35, 16.81)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning
Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	16.03 (14.890)	7.68 (20.541)	8.34 (23.654)	0.7408
	95% CI for LS MEAN	(-22.63, 54.68)	(-47.18, 62.55)	(-55.36, 72.04)	
Week 4	LS Mean (SEM)	16.03 (16.246)	-14.54 (22.813)	30.56 (26.462)	0.2960
	95% CI for LS MEAN	(-23.62, 55.67)	(-71.38, 42.31)	(-35.73, 96.86)	
Week 8	LS Mean (SEM)	30.94 (13.095)			
	95% CI for LS MEAN	(-4.04, 65.92)			
Week 12	LS Mean (SEM)	20.79 (10.910)	-31.20 (13.503)	51.99 (14.738)	0.0305
	95% CI for LS MEAN	(-10.18, 51.76)	(-70.21, 7.80)	(8.53, 95.45)	

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The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.9

Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Role Functioning

Subgroup by treatment interaction p-value	0.4961
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Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning
Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	4.73 (2.922)	0.59 (4.107)	4.14 (4.685)	0.3850
	95% CI for LS MEAN	(-1.25, 10.70)	(-7.81, 9.00)	(-5.46, 13.73)	
Week 4	LS Mean (SEM)	7.38 (2.863)	0.59 (4.015)	6.79 (4.568)	0.1486
	95% CI for LS MEAN	(1.52, 13.24)	(-7.63, 8.81)	(-2.57, 16.15)	
Week 8	LS Mean (SEM)	6.16 (3.465)	-0.24 (4.770)	6.40 (5.595)	0.2623
	95% CI for LS MEAN	(-0.90, 13.21)	(-9.99, 9.51)	(-5.05, 17.85)	
Week 12	LS Mean (SEM)	9.11 (3.109)	-0.24 (4.346)	9.35 (5.011)	0.0727
	95% CI for LS MEAN	(2.76, 15.46)	(-9.15, 8.66)	(-0.92, 19.63)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning
White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	7.07 (2.759)	-0.91 (4.719)	7.98 (5.452)	0.1536
	95% CI for LS MEAN	(1.43, 12.72)	(-10.54, 8.72)	(-3.15, 19.11)	
Week 4	LS Mean (SEM)	4.63 (3.056)	0.39 (5.116)	4.24 (5.947)	0.4809
	95% CI for LS MEAN	(-1.57, 10.83)	(-9.99, 10.78)	(-7.84, 16.31)	
Week 8	LS Mean (SEM)	5.62 (3.027)	9.93 (5.304)	-4.31 (6.094)	0.4864
	95% CI for LS MEAN	(-0.63, 11.87)	(-1.00, 20.86)	(-16.88, 8.27)	
Week 12	LS Mean (SEM)	6.89 (2.336)	4.56 (3.748)	2.33 (4.401)	0.6000
	95% CI for LS MEAN	(2.14, 11.64)	(-3.07, 12.19)	(-6.62, 11.28)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning
Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.56 (9.466)	1.48 (11.538)	4.07 (13.900)	0.7734
	95% CI for LS MEAN	(-15.00, 26.12)	(-23.25, 26.22)	(-25.53, 33.68)	
Week 4	LS Mean (SEM)	3.18 (9.466)	3.57 (11.538)	-0.39 (13.900)	0.9780
	95% CI for LS MEAN	(-17.39, 23.74)	(-21.17, 28.30)	(-30.00, 29.22)	
Week 8	LS Mean (SEM)	7.24 (10.735)	9.07 (20.606)	-1.83 (22.402)	0.9358
	95% CI for LS MEAN	(-15.40, 29.88)	(-34.57, 52.70)	(-48.71, 45.05)	
Week 12	LS Mean (SEM)	10.32 (9.466)	-17.27 (11.538)	27.59 (13.900)	0.0656
	95% CI for LS MEAN	(-10.24, 30.88)	(-42.00, 7.47)	(-2.02, 57.19)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Data cutoff date of 31Mar2023

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Table GERMANY.2.5.10.9

Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Emotional Functioning

Subgroup by treatment interaction p-value	0.7549
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Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning
Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	0.69 (3.385)	3.79 (4.866)	-3.10 (5.571)	0.5821
	95% CI for LS MEAN	(-6.24, 7.62)	(-6.17, 13.76)	(-14.52, 8.31)	
Week 4	LS Mean (SEM)	4.48 (3.735)	3.79 (5.401)	0.69 (6.247)	0.9133
	95% CI for LS MEAN	(-3.15, 12.11)	(-7.24, 14.83)	(-12.09, 13.47)	
Week 8	LS Mean (SEM)	4.74 (3.757)	3.79 (5.294)	0.95 (6.166)	0.8790
	95% CI for LS MEAN	(-2.92, 12.41)	(-7.03, 14.62)	(-11.67, 13.56)	
Week 12	LS Mean (SEM)	3.82 (3.757)	5.46 (5.328)	-1.64 (6.197)	0.7935
	95% CI for LS MEAN	(-3.83, 11.48)	(-5.43, 16.35)	(-14.31, 11.03)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning
White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	5.05 (2.835)	1.09 (4.890)	3.96 (5.640)	0.4870
	95% CI for LS MEAN	(-0.72, 10.82)	(-8.85, 11.02)	(-7.50, 15.43)	
Week 4	LS Mean (SEM)	6.87 (2.744)	-3.29 (4.591)	10.16 (5.337)	0.0655
	95% CI for LS MEAN	(1.29, 12.45)	(-12.62, 6.04)	(-0.69, 21.01)	
Week 8	LS Mean (SEM)	7.05 (3.098)	0.75 (5.349)	6.29 (6.169)	0.3156
	95% CI for LS MEAN	(0.72, 13.38)	(-10.15, 11.66)	(-6.29, 18.88)	
Week 12	LS Mean (SEM)	6.18 (2.360)	6.71 (3.775)	-0.53 (4.439)	0.9056
	95% CI for LS MEAN	(1.35, 11.01)	(-1.03, 14.44)	(-9.62, 8.56)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning
Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	17.69 (10.779)	-12.85 (12.656)	30.54 (15.080)	0.0696
	95% CI for LS MEAN	(-6.80, 42.18)	(-41.16, 15.45)	(-2.93, 64.01)	
Week 4	LS Mean (SEM)	8.16 (10.779)	-17.02 (12.656)	25.18 (15.080)	0.1250
	95% CI for LS MEAN	(-16.33, 32.65)	(-45.32, 11.28)	(-8.29, 58.66)	
Week 8	LS Mean (SEM)	14.26 (11.473)	0.09 (18.416)	14.16 (20.391)	0.4975
	95% CI for LS MEAN	(-11.29, 39.81)	(-39.16, 39.35)	(-29.16, 57.49)	
Week 12	LS Mean (SEM)	15.31 (10.779)	-21.19 (12.656)	36.49 (15.080)	0.0354
	95% CI for LS MEAN	(-9.18, 39.79)	(-49.49, 7.11)	(3.02, 69.97)	

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.9

Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Cognitive Functioning

Subgroup by treatment interaction p-value	0.0014
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EORTC QLQ-C30 is European Organization for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 Scale. Functional scales include global health status/QoL, physical functioning, role functioning, emotional functioning, cognitive functioning and social functioning.

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LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning
Asian

Visit	Statistics	Danicopan (N = 22)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.30 (3.976)	5.60 (5.702)	0.70 (6.648)	0.9170
	95% CI for LS MEAN	(-1.83, 14.43)	(-6.09, 17.29)	(-12.97, 14.36)	
Week 4	LS Mean (SEM)	8.57 (3.610)	12.27 (5.140)	-3.69 (5.944)	0.5394
	95% CI for LS MEAN	(1.19, 15.95)	(1.75, 22.79)	(-15.89, 8.50)	
Week 8	LS Mean (SEM)	8.73 (3.367)	5.60 (4.566)	3.13 (5.307)	0.5612
	95% CI for LS MEAN	(1.80, 15.66)	(-3.83, 15.03)	(-7.84, 14.10)	
Week 12	LS Mean (SEM)	9.84 (3.655)	5.60 (5.084)	4.24 (5.918)	0.4802
	95% CI for LS MEAN	(2.36, 17.32)	(-4.85, 16.05)	(-7.95, 16.44)	

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Each subscale has a range of 0 - 100%. A high scale score represents a higher response level. Thus a high score for a functional scale represents a high level of functioning but a high score for a symptom scale represents a high level of symptomatology/problem.

LS=Least squares; CI=Confidence Interval; SEM=Standard error of the LS mean.

The difference of LS Means is calculated as the Danicopan mean minus Placebo mean.

Estimates are based on an MMRM that includes fixed, categorical effects of treatment group, study visit, and study visit by treatment group interaction as well as the continuous, fixed covariate of baseline EORTC QLQ-C30 score value and the randomization stratification factors of transfusion history (> 2 or ≤ 2 transfusions within 6 months of Screening) and screening Hgb (< 8.5 g/dL or ≥ 8.5 g/dL).

P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning
White

Visit	Statistics	Danicopan (N = 28)	Placebo (N = 10)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	6.21 (3.511)	-0.70 (6.129)	6.91 (7.091)	0.3364
	95% CI for LS MEAN	(-0.92, 13.34)	(-13.11, 11.71)	(-7.46, 21.27)	
Week 4	LS Mean (SEM)	7.40 (2.563)	-6.67 (4.313)	14.06 (5.056)	0.0091
	95% CI for LS MEAN	(2.17, 12.63)	(-15.47, 2.13)	(3.75, 24.38)	
Week 8	LS Mean (SEM)	9.71 (4.301)	6.54 (7.551)	3.17 (8.708)	0.7179
	95% CI for LS MEAN	(0.94, 18.49)	(-8.83, 21.92)	(-14.56, 20.91)	
Week 12	LS Mean (SEM)	10.18 (3.156)	1.67 (5.088)	8.52 (6.015)	0.1680
	95% CI for LS MEAN	(3.72, 16.64)	(-8.77, 12.10)	(-3.81, 20.84)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.9
Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning
Others/Unknown

Visit	Statistics	Danicopan (N = 7)	Placebo (N = 4)	Difference (Danicopan-Placebo)	P-value
Week 2	LS Mean (SEM)	17.78 (9.194)	-24.06 (13.057)	41.84 (15.482)	0.0162
	95% CI for LS MEAN	(-2.41, 37.97)	(-51.88, 3.77)	(8.88, 74.79)	
Week 4	LS Mean (SEM)	13.02 (9.194)	-19.62 (12.301)	32.64 (14.682)	0.0411
	95% CI for LS MEAN	(-7.17, 33.21)	(-45.74, 6.49)	(1.49, 63.80)	
Week 8	LS Mean (SEM)	20.92 (9.688)			
	95% CI for LS MEAN	(-0.05, 41.89)			
Week 12	LS Mean (SEM)	15.40 (9.194)	-43.26 (11.091)	58.66 (13.466)	0.0007
	95% CI for LS MEAN	(-4.79, 35.59)	(-67.29, -19.23)	(29.66, 87.66)	

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P-value is from difference of LSM from MMRM model. Interaction test p-value is from the same MMRM model with additional race variable and race by treatment interaction term.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.10.9

Change from Baseline in EORTC QLQ-C30 Scores Functional Scales During 12-Week of Treatment Period 1 by Race
Analysis Using a Mixed Model for Repeated Measures (MMRM)
Modified Randomized Set

Social Functioning

Subgroup by treatment interaction p-value	0.0002
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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

3 Sicherheit

3.1 Unerwünschte Ereignisse

3.1.1 Unerwünschte Ereignisse innerhalb TP1

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Table GERMANY.3.1.3.1.1
Overview of Treatment-Emergent Adverse Events for Treatment Period 1
Safety Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value
Any Adverse Event (AE)	43 (75.4) (62.2, 85.9)	18 (62.1) (42.3, 79.3)	1.22 (0.90, 1.82)	1.88 (0.63, 5.44)	0.13 (-0.07, 0.35)	0.2175
Non-severe AE (CTCAE < 3)	42 (73.7) (60.3, 84.5)	18 (62.1) (42.3, 79.3)	1.19 (0.87, 1.79)	1.71 (0.58, 4.90)	0.12 (-0.09, 0.33)	0.3234
Any Serious AE (SAE)	3 (5.3) (1.1, 14.6)	2 (6.9) (0.8, 22.8)	0.76 (0.13, 7.61)	0.75 (0.08, 9.51)	-0.02 (-0.18, 0.10)	1.0000
Severe AE (CTCAE ≥ 3)	10 (17.5) (8.7, 29.9)	4 (13.8) (3.9, 31.7)	1.27 (0.45, 7.56)	1.33 (0.34, 6.39)	0.04 (-0.15, 0.19)	0.7646
AE Leading to Withdrawal of Study Drug	3 (5.3) (1.1, 14.6)	1 (3.4) (0.1, 17.8)	1.53 (0.16, 39.19)	1.56 (0.12, 84.54)	0.02 (-0.13, 0.12)	1.0000

TEAE = Treatment-emergent adverse event, % = n/N*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

P-value is from the fisher exact test for treatment comparison.

95% CI for each treatment group is computed using Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

Source: ADaM.ADSL, ADaM.ADAE

Run Date: 2024-04-26T15:35:23

Data cutoff date of 31Mar2023

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

3 Sicherheit

3.1 Unerwünschte Ereignisse

3.1.1 Unerwünschte Ereignisse innerhalb TP1

3.1.1.1 Subgruppenanalysen – Unerwünschte Ereignisse innerhalb TP1

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Table GERMANY.3.1.3.1.2
Overview of Treatment-Emergent Adverse Events for Treatment Period 1 by Sex
Safety Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Any Adverse Event (AE)							0.0031
Male (N=32)	m=23 19 (82.6) (61.2, 95.0)	m= 9 2 (22.2) (2.8, 60.0)	3.72 (1.27, 39.84)	16.63 (1.92, 197.39)	0.60 (0.14, 0.85)	0.0026	
Female (N=54)	m=34 24 (70.6) (52.5, 84.9)	m=20 16 (80.0) (56.3, 94.3)	0.88 (0.64, 1.32)	0.60 (0.12, 2.58)	-0.09 (-0.32, 0.17)	0.5326	

TEAE = Treatment-emergent adverse event, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The summary by subgroup is performed with at least 10 patients in each subgroup and at least 10 patients with the event in at least one subgroup.

Source: ADaM.ADSL, ADaM.ADAE

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:46:23

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Table GERMANY.3.1.3.1.2
Overview of Treatment-Emergent Adverse Events for Treatment Period 1 by Sex
Safety Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Non-severe AE (CTCAE < 3)							0.0019
Male (N=32)	m=23 19 (82.6) (61.2, 95.0)	m= 9 2 (22.2) (2.8, 60.0)	3.72 (1.27, 39.84)	16.63 (1.92, 197.39)	0.60 (0.14, 0.85)	0.0026	
Female (N=54)	m=34 23 (67.6) (49.5, 82.6)	m=20 16 (80.0) (56.3, 94.3)	0.85 (0.60, 1.26)	0.52 (0.10, 2.21)	-0.12 (-0.35, 0.15)	0.3675	

TEAE = Treatment-emergent adverse event, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The summary by subgroup is performed with at least 10 patients in each subgroup and at least 10 patients with the event in at least one subgroup.

Source: ADaM.ADSL, ADaM.ADAE

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:46:23

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Table GERMANY.3.1.3.1.2
Overview of Treatment-Emergent Adverse Events for Treatment Period 1 by Sex
Safety Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Severe AE (CTCAE ≥ 3)							NA
Male (N=32)	m=23 4 (17.4) (5.0, 38.8)	m= 9 0 (0.0, 33.6)	NA	NA	0.17 (-0.18, 0.39)	0.3035	
Female (N=54)	m=34 6 (17.6) (6.8, 34.5)	m=20 4 (20.0) (5.7, 43.7)	0.88 (0.27, 3.44)	0.86 (0.17, 4.79)	-0.02 (-0.28, 0.19)	1.0000	

TEAE = Treatment-emergent adverse event, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The summary by subgroup is performed with at least 10 patients in each subgroup and at least 10 patients with the event in at least one subgroup.

Source: ADaM.ADSL, ADaM.ADAE

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:46:23

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Table GERMANY.3.1.3.1.3
Overview of Treatment-Emergent Adverse Events for Treatment Period 1 by Age at Informed Consent
(< 65 and ≥ 65 Years)
Safety Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Any Adverse Event (AE)							0.8960
<65 (N=64)	m=41 32 (78.0) (62.4, 89.4)	m=23 15 (65.2) (42.7, 83.6)	1.20 (0.87, 1.88)	1.90 (0.52, 6.78)	0.13 (-0.11, 0.37)	0.3768	
≥65 (N=22)	m=16 11 (68.8) (41.3, 89.0)	m= 6 3 (50.0) (11.8, 88.2)	1.38 (0.65, 5.46)	2.20 (0.21, 22.33)	0.19 (-0.27, 0.63)	0.6244	

TEAE = Treatment-emergent adverse event, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The summary by subgroup is performed with at least 10 patients in each subgroup and at least 10 patients with the event in at least one subgroup.

Source: ADaM.ADSL, ADaM.ADAE

Data cutoff date of 31Mar2023

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Table GERMANY.3.1.3.1.3
Overview of Treatment-Emergent Adverse Events for Treatment Period 1 by Age at Informed Consent
(< 65 and ≥ 65 Years)
Safety Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Non-severe AE (CTCAE < 3)							0.8007
<65 (N=64)	m=41 31 (75.6) (59.7, 87.6)	m=23 15 (65.2) (42.7, 83.6)	1.16 (0.84, 1.85)	1.65 (0.46, 5.77)	0.10 (-0.13, 0.35)	0.3989	
≥65 (N=22)	m=16 11 (68.8) (41.3, 89.0)	m= 6 3 (50.0) (11.8, 88.2)	1.38 (0.65, 5.46)	2.20 (0.21, 22.33)	0.19 (-0.27, 0.63)	0.6244	

TEAE = Treatment-emergent adverse event, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The summary by subgroup is performed with at least 10 patients in each subgroup and at least 10 patients with the event in at least one subgroup.

Source: ADaM.ADSL, ADaM.ADAE

Data cutoff date of 31Mar2023

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Table GERMANY.3.1.3.1.3
Overview of Treatment-Emergent Adverse Events for Treatment Period 1 by Age at Informed Consent
(< 65 and ≥ 65 Years)
Safety Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Severe AE (CTCAE ≥ 3)							0.5872
<65 (N=64)	m=41 8 (19.5) (8.8, 34.9)	m=23 3 (13.0) (2.8, 33.6)	1.50 (0.47, 8.36)	1.62 (0.33, 10.48)	0.06 (-0.16, 0.25)	0.7323	
≥65 (N=22)	m=16 2 (12.5) (1.6, 38.3)	m= 6 1 (16.7) (0.4, 64.1)	0.75 (0.07, 20.30)	0.71 (0.03, 50.47)	-0.04 (-0.52, 0.28)	1.0000	

TEAE = Treatment-emergent adverse event, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The summary by subgroup is performed with at least 10 patients in each subgroup and at least 10 patients with the event in at least one subgroup.

Source: ADaM.ADSL, ADaM.ADAE

Data cutoff date of 31Mar2023

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Table GERMANY.3.1.3.1.4
Overview of Treatment-Emergent Adverse Events for Treatment Period 1 by Region
Safety Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Any Adverse Event (AE)							0.2114
Europe (N=41)	m=25 22 (88.0) (68.8, 97.5)	m=16 10 (62.5) (35.4, 84.8)	1.41 (0.96, 2.59)	4.40 (0.73, 31.59)	0.26 (-0.03, 0.54)	0.1185	
Rest of World (N=45)	m=32 21 (65.6) (46.8, 81.4)	m=13 8 (61.5) (31.6, 86.1)	1.07 (0.67, 2.30)	1.19 (0.24, 5.39)	0.04 (-0.26, 0.36)	1.0000	

TEAE = Treatment-emergent adverse event, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The summary by subgroup is performed with at least 10 patients in each subgroup and at least 10 patients with the event in at least one subgroup.

Source: ADaM.ADSL, ADaM.ADAE

Data cutoff date of 31Mar2023

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Table GERMANY.3.1.3.1.4
Overview of Treatment-Emergent Adverse Events for Treatment Period 1 by Region
Safety Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Non-severe AE (CTCAE < 3)							0.3363
Europe (N=41)	m=25 21 (84.0) (63.9, 95.5)	m=16 10 (62.5) (35.4, 84.8)	1.34 (0.90, 2.45)	3.15 (0.57, 18.39)	0.22 (-0.07, 0.50)	0.1496	
Rest of World (N=45)	m=32 21 (65.6) (46.8, 81.4)	m=13 8 (61.5) (31.6, 86.1)	1.07 (0.67, 2.30)	1.19 (0.24, 5.39)	0.04 (-0.26, 0.36)	1.0000	

TEAE = Treatment-emergent adverse event, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The summary by subgroup is performed with at least 10 patients in each subgroup and at least 10 patients with the event in at least one subgroup.

Source: ADaM.ADSL, ADaM.ADAE

Data cutoff date of 31Mar2023

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Table GERMANY.3.1.3.1.5
Overview of Treatment-Emergent Adverse Events for Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Safety Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Any Adverse Event (AE)							0.0789
≤2 (N=50)	m=33 27 (81.8) (64.5, 93.0)	m=17 9 (52.9) (27.8, 77.0)	1.55 (1.00, 3.21)	4.00 (0.90, 17.93)	0.29 (0.00, 0.56)	0.0470	
>2 (N=36)	m=24 16 (66.7) (44.7, 84.4)	m=12 9 (75.0) (42.8, 94.5)	0.89 (0.56, 1.59)	0.67 (0.09, 3.83)	-0.08 (-0.38, 0.27)	0.7148	

TEAE = Treatment-emergent adverse event, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The summary by subgroup is performed with at least 10 patients in each subgroup and at least 10 patients with the event in at least one subgroup.

Source: ADaM.ADSL, ADaM.ADAE

Data cutoff date of 31Mar2023

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Table GERMANY.3.1.3.1.5
Overview of Treatment-Emergent Adverse Events for Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Safety Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Non-severe AE (CTCAE < 3)							0.0511
≤2 (N=50)	m=33 27 (81.8) (64.5, 93.0)	m=17 9 (52.9) (27.8, 77.0)	1.55 (1.00, 3.21)	4.00 (0.90, 17.93)	0.29 (0.00, 0.56)	0.0470	
>2 (N=36)	m=24 15 (62.5) (40.6, 81.2)	m=12 9 (75.0) (42.8, 94.5)	0.83 (0.52, 1.56)	0.56 (0.08, 3.13)	-0.13 (-0.42, 0.23)	0.7091	

TEAE = Treatment-emergent adverse event, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The summary by subgroup is performed with at least 10 patients in each subgroup and at least 10 patients with the event in at least one subgroup.

Source: ADaM.ADSL, ADaM.ADAE

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:47:10

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Table GERMANY.3.1.3.1.6
Overview of Treatment-Emergent Adverse Events for Treatment Period 1 by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Safety Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Any Adverse Event (AE)							0.2363
<8.5 g/dL (N=52)	m=35 27 (77.1) (59.9, 89.6)	m=17 9 (52.9) (27.8, 77.0)	1.46 (0.95, 3.06)	3.00 (0.73, 12.26)	0.24 (-0.04, 0.52)	0.1106	
≥8.5 g/dL (N=34)	m=22 16 (72.7) (49.8, 89.3)	m=12 9 (75.0) (42.8, 94.5)	0.97 (0.63, 1.73)	0.89 (0.12, 5.53)	-0.02 (-0.32, 0.33)	1.0000	

TEAE = Treatment-emergent adverse event, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The summary by subgroup is performed with at least 10 patients in each subgroup and at least 10 patients with the event in at least one subgroup.

Source: ADaM.ADSL, ADaM.ADAE

Data cutoff date of 31Mar2023

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Table GERMANY.3.1.3.1.6
Overview of Treatment-Emergent Adverse Events for Treatment Period 1 by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Safety Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Non-severe AE (CTCAE < 3)							0.2997
<8.5 g/dL (N=52)	m=35 26 (74.3) (56.7, 87.5)	m=17 9 (52.9) (27.8, 77.0)	1.40 (0.90, 2.89)	2.57 (0.64, 10.22)	0.21 (-0.07, 0.49)	0.2066	
≥8.5 g/dL (N=34)	m=22 16 (72.7) (49.8, 89.3)	m=12 9 (75.0) (42.8, 94.5)	0.97 (0.63, 1.73)	0.89 (0.12, 5.53)	-0.02 (-0.32, 0.33)	1.0000	

TEAE = Treatment-emergent adverse event, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The summary by subgroup is performed with at least 10 patients in each subgroup and at least 10 patients with the event in at least one subgroup.

Source: ADaM.ADSL, ADaM.ADAE

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:47:21

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Table GERMANY.3.1.3.1.7
Overview of Treatment-Emergent Adverse Events for Treatment Period 1 by Background C5 Inhibitor Therapy
(Eculizumab or Ravulizumab)
Safety Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Any Adverse Event (AE)							0.4935
Eculizumab (N=35)	m=21 19 (90.5) (69.6, 98.8)	m=14 10 (71.4) (41.9, 91.6)	1.27 (0.90, 2.14)	3.80 (0.44, 47.00)	0.19 (-0.08, 0.49)	0.1907	
Ravulizumab (N=51)	m=36 24 (66.7) (49.0, 81.4)	m=15 8 (53.3) (26.6, 78.7)	1.25 (0.77, 2.92)	1.75 (0.42, 7.05)	0.13 (-0.16, 0.42)	0.5261	

TEAE = Treatment-emergent adverse event, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The summary by subgroup is performed with at least 10 patients in each subgroup and at least 10 patients with the event in at least one subgroup.

Source: ADaM.ADSL, ADaM.ADAE

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:47:32

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Table GERMANY.3.1.3.1.7
Overview of Treatment-Emergent Adverse Events for Treatment Period 1 by Background C5 Inhibitor Therapy
(Eculizumab or Ravulizumab)
Safety Set

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
Non-severe AE (CTCAE < 3)							0.7664
Eculizumab (N=35)	m=21 18 (85.7) (63.7, 97.0)	m=14 10 (71.4) (41.9, 91.6)	1.20 (0.83, 2.09)	2.40 (0.32, 19.34)	0.14 (-0.16, 0.45)	0.4007	
Ravulizumab (N=51)	m=36 24 (66.7) (49.0, 81.4)	m=15 8 (53.3) (26.6, 78.7)	1.25 (0.77, 2.92)	1.75 (0.42, 7.05)	0.13 (-0.16, 0.42)	0.5261	

TEAE = Treatment-emergent adverse event, m = number of patients in each subgroup and treatment group, % = n/m*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The summary by subgroup is performed with at least 10 patients in each subgroup and at least 10 patients with the event in at least one subgroup.

Source: ADaM.ADSL, ADaM.ADAE

Data cutoff date of 31Mar2023

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

3 Sicherheit

3.1 Unerwünschte Ereignisse

3.1.2 Unerwünschte Ereignisse innerhalb der gesamten Behandlungsdauer (inkl. TP2 und LTE)

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Table GERMANY.3.1.3.1.8
Overview of Treatment-Emergent Adverse Events for Overall Danicopan Treatment Up to Data Cut-off
Safety Set

	D->D (N=57) n (%) (95% CI)	P->D (N=27) n (%) (95% CI)	Total (N=84) n (%) (95% CI)
Any Adverse Event (AE)	55 (96.5) (87.9, 99.6)	25 (92.6) (75.7, 99.1)	80 (95.2) (88.3, 98.7)
Non-severe AE (CTCAE < 3)	53 (93.0) (83.0, 98.1)	24 (88.9) (70.8, 97.6)	77 (91.7) (83.6, 96.6)
Any Serious AE (SAE)	9 (15.8) (7.5, 27.9)	8 (29.6) (13.8, 50.2)	17 (20.2) (12.3, 30.4)
Severe AE (CTCAE ≥ 3)	19 (33.3) (21.4, 47.1)	10 (37.0) (19.4, 57.6)	29 (34.5) (24.5, 45.7)
AE Leading to Withdrawal of Study Drug	3 (5.3) (1.1, 14.6)	2 (7.4) (0.9, 24.3)	5 (6.0) (2.0, 13.3)
Death	0 (0.0, 6.3)	0 (0.0, 12.8)	0 (0.0, 4.3)

TEAE = Treatment-emergent adverse event, % = n/N*100, CI=Confidence Interval

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

95% CI for each treatment group is computed using Clopper-Pearson method.

Source: ADaM.ADSL, ADaM.ADAE

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Data cutoff date of 31Mar2023

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

3 Sicherheit

3.2 Unerwünschte Ereignisse nach SOC/PT

3.2.1 Unerwünschte Ereignisse nach SOC/PT innerhalb TP1

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Table GERMANY.3.1.3.2.1.1
Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term for Treatment Period 1
Safety Set

System Organ Class (SOC) Preferred Term (PT)	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value
Any Adverse Event	43 (75.4) (62.2, 85.9)	18 (62.1) (42.3, 79.3)	1.22 (0.90, 1.82)	1.88 (0.63, 5.44)	0.13 (-0.07, 0.35)	0.2175
Blood and lymphatic system disorders	5 (8.8) (2.9, 19.3)	4 (13.8) (3.9, 31.7)	0.64 (0.16, 2.69)	0.60 (0.12, 3.32)	-0.05 (-0.24, 0.09)	0.4782
Anaemia	1 (1.8) (0.0, 9.4)	4 (13.8) (3.9, 31.7)	0.13 (0.01, 0.90)	0.11 (0.00, 1.23)	-0.12 (-0.30, -0.01)	0.0423
Gastrointestinal disorders	17 (29.8) (18.4, 43.4)	9 (31.0) (15.3, 50.8)	0.96 (0.49, 2.17)	0.94 (0.33, 2.86)	-0.01 (-0.23, 0.19)	1.0000
Nausea	5 (8.8) (2.9, 19.3)	3 (10.3) (2.2, 27.4)	0.85 (0.20, 7.42)	0.83 (0.15, 5.79)	-0.02 (-0.19, 0.11)	1.0000
Diarrhoea	4 (7.0) (1.9, 17.0)	3 (10.3) (2.2, 27.4)	0.68 (0.15, 4.93)	0.65 (0.10, 4.82)	-0.03 (-0.21, 0.09)	0.6836

% = n/N*100, RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

SOCs and PTs are sorted alphabetically.

SOCs and PTs were coded using MedDRA version 25.1.

P-value is from the fisher exact test for treatment comparison. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

SOCs and PTs with at least 10% patients in a study arm, or in 10 patients in one treatment arm is summarized.

Source: ADaM.ADSL, ADaM.ADAE

Run Date: 2024-04-26T15:44:50

Data cutoff date of 31Mar2023

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Table GERMANY.3.1.3.2.1.1
Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term for Treatment Period 1
Safety Set

System Organ Class (SOC) Preferred Term (PT)	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value
General disorders and administration site conditions	6 (10.5) (4.0, 21.5)	6 (20.7) (8.0, 39.7)	0.51 (0.16, 1.63)	0.45 (0.11, 1.90)	-0.10 (-0.30, 0.06)	0.2083
Asthenia	0 (0.0, 6.3)	4 (13.8) (3.9, 31.7)	0.00 (0.00, 0.52)	0.00 (0.00, 0.53)	-0.14 (-0.32, -0.04)	0.0112
Infections and infestations	11 (19.3) (10.0, 31.9)	7 (24.1) (10.3, 43.5)	0.80 (0.34, 2.09)	0.75 (0.23, 2.63)	-0.05 (-0.26, 0.13)	0.5893
Injury, poisoning and procedural complications	8 (14.0) (6.3, 25.8)	3 (10.3) (2.2, 27.4)	1.36 (0.41, 7.61)	1.41 (0.30, 8.95)	0.04 (-0.15, 0.18)	0.7433
Contusion	2 (3.5) (0.4, 12.1)	3 (10.3) (2.2, 27.4)	0.34 (0.03, 2.03)	0.32 (0.03, 2.97)	-0.07 (-0.24, 0.05)	0.3300
Investigations	8 (14.0) (6.3, 25.8)	4 (13.8) (3.9, 31.7)	1.02 (0.32, 6.22)	1.02 (0.24, 5.09)	0.00 (-0.19, 0.15)	1.0000
Aspartate aminotransferase increased	2 (3.5) (0.4, 12.1)	3 (10.3) (2.2, 27.4)	0.34 (0.03, 2.03)	0.32 (0.03, 2.97)	-0.07 (-0.24, 0.05)	0.3300

% = n/N*100, RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

SOCs and PTs are sorted alphabetically.

SOCs and PTs were coded using MedDRA version 25.1.

P-value is from the fisher exact test for treatment comparison. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

SOCs and PTs with at least 10% patients in a study arm, or in 10 patients in one treatment arm is summarized.

Source: ADaM.ADSL, ADaM.ADAE

Run Date: 2024-04-26T15:44:50

Data cutoff date of 31Mar2023

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Table GERMANY.3.1.3.2.1.1
Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term for Treatment Period 1
Safety Set

System Organ Class (SOC) Preferred Term (PT)	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value
Musculoskeletal and connective tissue disorders	10 (17.5) (8.7, 29.9)	5 (17.2) (5.8, 35.8)	1.02 (0.39, 3.84)	1.02 (0.28, 4.25)	0.00 (-0.19, 0.17)	1.0000
Nervous system disorders	7 (12.3) (5.1, 23.7)	5 (17.2) (5.8, 35.8)	0.71 (0.24, 2.24)	0.67 (0.16, 2.99)	-0.05 (-0.25, 0.11)	0.5283
Headache	6 (10.5) (4.0, 21.5)	3 (10.3) (2.2, 27.4)	1.02 (0.27, 7.56)	1.02 (0.20, 6.80)	0.00 (-0.18, 0.14)	1.0000
Psychiatric disorders	2 (3.5) (0.4, 12.1)	3 (10.3) (2.2, 27.4)	0.34 (0.03, 2.03)	0.32 (0.03, 2.97)	-0.07 (-0.24, 0.05)	0.3300
Insomnia	1 (1.8) (0.0, 9.4)	3 (10.3) (2.2, 27.4)	0.17 (0.01, 1.63)	0.15 (0.00, 2.08)	-0.09 (-0.26, 0.02)	0.1093
Respiratory, thoracic and mediastinal disorders	7 (12.3) (5.1, 23.7)	2 (6.9) (0.8, 22.8)	1.78 (0.42, 16.97)	1.89 (0.33, 19.76)	0.05 (-0.11, 0.18)	0.7117
Vascular disorders	5 (8.8) (2.9, 19.3)	3 (10.3) (2.2, 27.4)	0.85 (0.20, 7.42)	0.83 (0.15, 5.79)	-0.02 (-0.19, 0.11)	1.0000

% = n/N*100, RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

SOCs and PTs are sorted alphabetically.

SOCs and PTs were coded using MedDRA version 25.1.

P-value is from the fisher exact test for treatment comparison. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

SOCs and PTs with at least 10% patients in a study arm, or in 10 patients in one treatment arm is summarized.

Source: ADaM.ADSL, ADaM.ADAE

Run Date: 2024-04-26T15:44:50

Data cutoff date of 31Mar2023

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Table GERMANY.3.1.3.2.2.1
Severe Treatment-Emergent Adverse Events (CTCAE \geq 3) by MedDRA System Organ Class and Preferred Term for Treatment Period 1
Safety Set

System Organ Class (SOC) Preferred Term (PT)	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value
Any Adverse Event	10 (17.5) (8.7, 29.9)	4 (13.8) (3.9, 31.7)	1.27 (0.45, 7.56)	1.33 (0.34, 6.39)	0.04 (-0.15, 0.19)	0.7646
Blood and lymphatic system disorders	2 (3.5) (0.4, 12.1)	3 (10.3) (2.2, 27.4)	0.34 (0.03, 2.03)	0.32 (0.03, 2.97)	-0.07 (-0.24, 0.05)	0.3300
Anaemia	1 (1.8) (0.0, 9.4)	2 (6.9) (0.8, 22.8)	0.25 (0.01, 2.78)	0.24 (0.00, 4.91)	-0.05 (-0.21, 0.04)	0.2618
Investigations	6 (10.5) (4.0, 21.5)	0 (0.0, 11.9)	NA	NA	0.11 (-0.03, 0.22)	0.0926
Alanine aminotransferase increased	3 (5.3) (1.1, 14.6)	0 (0.0, 11.9)	NA	NA	0.05 (-0.08, 0.15)	0.5477

% = n/N*100, RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

SOCs and PTs are sorted alphabetically.

SOCs and PTs were coded using MedDRA version 25.1.

P-value is from the fisher exact test for treatment comparison. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

SOCs and PTs with at least 5% patients in a study arm, or in 10 patients in one treatment arm is summarized.

Source: ADaM.ADSL, ADaM.ADAE

Run Date: 2024-04-26T15:46:07

Data cutoff date of 31Mar2023

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Table GERMANY.3.1.3.2.3.1
Serious Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term for Treatment Period 1
Safety Set

System Organ Class (SOC) Preferred Term (PT)	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value
Any Adverse Event	3 (5.3) (1.1, 14.6)	2 (6.9) (0.8, 22.8)	0.76 (0.13, 7.61)	0.75 (0.08, 9.51)	-0.02 (-0.18, 0.10)	1.0000

% = n/N*100, RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

SOCs and PTs are sorted alphabetically.

SOCs and PTs were coded using MedDRA version 25.1.

P-value is from the fisher exact test for treatment comparison. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

SOCs and PTs with at least 5% patients in a study arm, or in 10 patients in one treatment arm is summarized.

Source: ADaM.ADSL, ADaM.ADAE

Run Date: 2024-04-26T15:46:20

Data cutoff date of 31Mar2023

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Table GERMANY.3.1.3.2.4.1
Treatment-Emergent Adverse Events Leading to Withdrawal of Study Drug by MedDRA System Organ Class and Preferred Term
for Treatment Period 1
Safety Set

System Organ Class (SOC) Preferred Term (PT)	Danicopan (N=57) n (%)	Placebo (N=29) n (%)
Any Adverse Event	3 (5.3)	1 (3.4)
Gastrointestinal disorders	1 (1.8)	0
Pancreatitis	1 (1.8)	0
Investigations	3 (5.3)	1 (3.4)
Alanine aminotransferase increased	1 (1.8)	0
Aspartate aminotransferase increased	1 (1.8)	1 (3.4)
Blood bilirubin increased	1 (1.8)	0
Hepatic enzyme increased	1 (1.8)	0

% = n/N*100.

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

SOCs are sorted alphabetically and PTs are sorted by decreasing percent based on Danicopan column.

SOCs and PTs were coded using MedDRA version 25.1.

Source: ADaM.ADSL, ADaM.ADAE

Run Date: 2024-04-26T15:46:20

Data cutoff date of 31Mar2023

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

3 Sicherheit

3.2 Unerwünschte Ereignisse nach SOC/PT

3.2.1 Unerwünschte Ereignisse nach SOC/PT innerhalb TP1

3.2.1.1 Subgruppenanalysen – Unerwünschte Ereignisse nach SOC/PT innerhalb TP1

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Table GERMANY.3.1.3.2.1.2
Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term for Treatment Period 1 by Sex
Safety Set

	Danicopan (N=57)	Placebo (N=29)					B-D test
System Organ Class (SOC)	n (%)	n (%)	RR	OR	RD	p-value	p-value
Preferred Term (PT)	(95% CI)	(95% CI)	(95% CI)	(95% CI)	(95% CI)	[a]	[b]

No data match the criteria for this report.

m = number of patients in each subgroup and treatment group, % = n/m*100, RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

SOCs and PTs are sorted alphabetically. SOC and PTs were coded using MedDRA version 25.1.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients is summarized.

SOCs and PTs with at least 10% patients in a study arm, or in 10 patients in one treatment arm is summarized. Only SOC and PTs in Table Germany.3.1.3.2.1.1 with p-value < 0.05 are presented in this table.

Source: ADaM.ADSL, ADaM.ADAE

Data cutoff date of 31Mar2023

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Table GERMANY.3.1.3.2.1.3
Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term for Treatment Period 1 by Age
at Informed Consent (< 65 and ≥ 65 Years)
Safety Set

	Danicopan (N=57)	Placebo (N=29)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
System Organ Class (SOC) Preferred Term (PT)	n (%) (95% CI)	n (%) (95% CI)					

No data match the criteria for this report.

m = number of patients in each subgroup and treatment group, % = n/m*100, RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

SOCs and PTs are sorted alphabetically. SOC and PTs were coded using MedDRA version 25.1.

[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

The subgroup with at least 10 patients is summarized.

SOCs and PTs with at least 10% patients in a study arm, or in 10 patients in one treatment arm is summarized. Only SOC and PTs in Table Germany.3.1.3.2.1.1 with p-value < 0.05 are presented in this table.

Source: ADaM.ADSL, ADaM.ADAE

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:42:11

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Table GERMANY.3.1.3.2.1.4
Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term for Treatment Period 1 by Region
Safety Set

	Danicopan (N=57)	Placebo (N=29)					B-D test
System Organ Class (SOC)	n (%)	n (%)	RR	OR	RD	p-value	p-value
Preferred Term (PT)	(95% CI)	(95% CI)	(95% CI)	(95% CI)	(95% CI)	[a]	[b]

No data match the criteria for this report.

m = number of patients in each subgroup and treatment group, % = n/m*100, RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.
 In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.
 TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.
 SOCs and PTs are sorted alphabetically. SOCs and PTs were coded using MedDRA version 25.1.
 [a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup.
 The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.
 The subgroup with at least 10 patients is summarized.
 SOCs and PTs with at least 10% patients in a study arm, or in 10 patients in one treatment arm is summarized. Only SOCs and PTs in Table Germany.3.1.3.2.1.1 with p-value < 0.05 are presented in this table.
 Source: ADaM.ADSL, ADaM.ADAE
 Run Date: 2024-04-26T15:42:11
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Table GERMANY.3.1.3.2.1.5
Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term for Treatment Period 1 by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Safety Set

	Danicopan (N=57)	Placebo (N=29)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
System Organ Class (SOC)	n (%)	n (%)					
Preferred Term (PT)	(95% CI)	(95% CI)					

No data match the criteria for this report.

m = number of patients in each subgroup and treatment group, % = n/m*100, RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.
 In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.
 TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.
 SOCs and PTs are sorted alphabetically. SOCs and PTs were coded using MedDRA version 25.1.
 [a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup.
 The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.
 The subgroup with at least 10 patients is summarized.
 SOCs and PTs with at least 10% patients in a study arm, or in 10 patients in one treatment arm is summarized. Only SOCs and PTs in Table Germany.3.1.3.2.1.1 with p-value < 0.05 are presented in this table.
 Source: ADaM.ADSL, ADaM.ADAE
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Table GERMANY.3.1.3.2.1.6
Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term for Treatment Period 1 by the
Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Safety Set

	Danicopan (N=57)	Placebo (N=29)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
System Organ Class (SOC) Preferred Term (PT)	n (%) (95% CI)	n (%) (95% CI)					

No data match the criteria for this report.

m = number of patients in each subgroup and treatment group, % = n/m*100, RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.
In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.
TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.
SOCs and PTs are sorted alphabetically. SOCs and PTs were coded using MedDRA version 25.1.
[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup.
The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.
The subgroup with at least 10 patients is summarized.
SOCs and PTs with at least 10% patients in a study arm, or in 10 patients in one treatment arm is summarized. Only SOCs and PTs in Table Germany.3.1.3.2.1.1 with p-value < 0.05 are presented in this table.
Source: ADaM.ADSL, ADaM.ADAE
Run Date: 2024-04-26T15:42:12
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Table GERMANY.3.1.3.2.1.7
Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term for Treatment Period 1
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Safety Set

	Danicopan (N=57)	Placebo (N=29)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value [a]	B-D test p-value [b]
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No data match the criteria for this report.

m = number of patients in each subgroup and treatment group, % = n/m*100, RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.
In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.
TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.
SOCs and PTs are sorted alphabetically. SOCs and PTs were coded using MedDRA version 25.1.
[a] P-value is from the fisher exact test for treatment comparison. [b] P-value is from the Breslow-Day test of homogeneity of odds ratios for subgroup.
The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.
The subgroup with at least 10 patients is summarized.
SOCs and PTs with at least 10% patients in a study arm, or in 10 patients in one treatment arm is summarized. Only SOCs and PTs in Table Germany.3.1.3.2.1.1 with p-value < 0.05 are presented in this table.
Source: ADaM.ADSL, ADaM.ADAE
Run Date: 2024-04-26T15:42:12
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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

3 Sicherheit

3.2 Unerwünschte Ereignisse nach SOC/PT

3.2.2 Unerwünschte Ereignisse nach SOC/PT innerhalb der gesamten Behandlungsdauer (inkl. TP2 und LTE)

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Table GERMANY.3.1.3.2.1.8
Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term for Overall Danicopan Treatment
Up to Data Cut-off
Safety Set

System Organ Class (SOC) Preferred Term (PT)	D->D (N=57) n (%) (95% CI)	P->D (N=27) n (%) (95% CI)	Total (N=84) n (%) (95% CI)
Any Adverse Event	55 (96.5) (87.9, 99.6)	25 (92.6) (75.7, 99.1)	80 (95.2) (88.3, 98.7)
Blood and lymphatic system disorders	14 (24.6) (14.1, 37.8)	8 (29.6) (13.8, 50.2)	22 (26.2) (17.2, 36.9)
Haemolysis	3 (5.3) (1.1, 14.6)	3 (11.1) (2.4, 29.2)	6 (7.1) (2.7, 14.9)
Cardiac disorders	3 (5.3) (1.1, 14.6)	4 (14.8) (4.2, 33.7)	7 (8.3) (3.4, 16.4)
Ear and labyrinth disorders	2 (3.5) (0.4, 12.1)	3 (11.1) (2.4, 29.2)	5 (6.0) (2.0, 13.3)
Eye disorders	3 (5.3) (1.1, 14.6)	3 (11.1) (2.4, 29.2)	6 (7.1) (2.7, 14.9)

% = n/N*100, CI=Confidence Interval

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period are regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods is only counted once in the treatment period the event started.

SOCs and PTs are sorted alphabetically.

SOCs and PTs were coded using MedDRA version 25.1.

The 95% CI for each treatment group is computed by Clopper-Pearson method.

SOCs and PTs with at least 10% patients in a study arm, or in 10 patients in one treatment arm is summarized.

Source: ADaM.ADSL, ADaM.ADAE

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Table GERMANY.3.1.3.2.1.8
Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term for Overall Danicopan Treatment
Up to Data Cut-off
Safety Set

System Organ Class (SOC) Preferred Term (PT)	D->D (N=57) n (%) (95% CI)	P->D (N=27) n (%) (95% CI)	Total (N=84) n (%) (95% CI)
Gastrointestinal disorders	27 (47.4) (34.0, 61.0)	12 (44.4) (25.5, 64.7)	39 (46.4) (35.5, 57.6)
Abdominal pain	1 (1.8) (0.0, 9.4)	3 (11.1) (2.4, 29.2)	4 (4.8) (1.3, 11.7)
Constipation	3 (5.3) (1.1, 14.6)	3 (11.1) (2.4, 29.2)	6 (7.1) (2.7, 14.9)
Diarrhoea	8 (14.0) (6.3, 25.8)	4 (14.8) (4.2, 33.7)	12 (14.3) (7.6, 23.6)
Dyspepsia	1 (1.8) (0.0, 9.4)	3 (11.1) (2.4, 29.2)	4 (4.8) (1.3, 11.7)
Nausea	7 (12.3) (5.1, 23.7)	3 (11.1) (2.4, 29.2)	10 (11.9) (5.9, 20.8)

% = n/N*100, CI=Confidence Interval

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period are regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods is only counted once in the treatment period the event started.

SOCs and PTs are sorted alphabetically.

SOCs and PTs were coded using MedDRA version 25.1.

The 95% CI for each treatment group is computed by Clopper-Pearson method.

SOCs and PTs with at least 10% patients in a study arm, or in 10 patients in one treatment arm is summarized.

Source: ADaM.ADSL, ADaM.ADAE

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Table GERMANY.3.1.3.2.1.8
Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term for Overall Danicopan Treatment
Up to Data Cut-off
Safety Set

System Organ Class (SOC) Preferred Term (PT)	D->D (N=57) n (%) (95% CI)	P->D (N=27) n (%) (95% CI)	Total (N=84) n (%) (95% CI)
General disorders and administration site conditions	27 (47.4) (34.0, 61.0)	11 (40.7) (22.4, 61.2)	38 (45.2) (34.3, 56.5)
Asthenia	4 (7.0) (1.9, 17.0)	4 (14.8) (4.2, 33.7)	8 (9.5) (4.2, 17.9)
Fatigue	7 (12.3) (5.1, 23.7)	2 (7.4) (0.9, 24.3)	9 (10.7) (5.0, 19.4)
Pyrexia	16 (28.1) (17.0, 41.5)	3 (11.1) (2.4, 29.2)	19 (22.6) (14.2, 33.0)
Hepatobiliary disorders	8 (14.0) (6.3, 25.8)	5 (18.5) (6.3, 38.1)	13 (15.5) (8.5, 25.0)

% = n/N*100, CI=Confidence Interval

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period are regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods is only counted once in the treatment period the event started.

SOCs and PTs are sorted alphabetically.

SOCs and PTs were coded using MedDRA version 25.1.

The 95% CI for each treatment group is computed by Clopper-Pearson method.

SOCs and PTs with at least 10% patients in a study arm, or in 10 patients in one treatment arm is summarized.

Source: ADaM.ADSL, ADaM.ADAE

Run Date: 2024-04-26T15:39:31

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Table GERMANY.3.1.3.2.1.8
Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term for Overall Danicopan Treatment
Up to Data Cut-off
Safety Set

System Organ Class (SOC) Preferred Term (PT)	D->D (N=57) n (%) (95% CI)	P->D (N=27) n (%) (95% CI)	Total (N=84) n (%) (95% CI)
Infections and infestations	31 (54.4) (40.7, 67.6)	19 (70.4) (49.8, 86.2)	50 (59.5) (48.3, 70.1)
COVID-19	14 (24.6) (14.1, 37.8)	10 (37.0) (19.4, 57.6)	24 (28.6) (19.2, 39.5)
Urinary tract infection	6 (10.5) (4.0, 21.5)	2 (7.4) (0.9, 24.3)	8 (9.5) (4.2, 17.9)
Injury, poisoning and procedural complications	10 (17.5) (8.7, 29.9)	6 (22.2) (8.6, 42.3)	16 (19.0) (11.3, 29.1)
Investigations	18 (31.6) (19.9, 45.2)	5 (18.5) (6.3, 38.1)	23 (27.4) (18.2, 38.2)
Metabolism and nutrition disorders	8 (14.0) (6.3, 25.8)	2 (7.4) (0.9, 24.3)	10 (11.9) (5.9, 20.8)

% = n/N*100, CI=Confidence Interval

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period are regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods is only counted once in the treatment period the event started.

SOCs and PTs are sorted alphabetically.

SOCs and PTs were coded using MedDRA version 25.1.

The 95% CI for each treatment group is computed by Clopper-Pearson method.

SOCs and PTs with at least 10% patients in a study arm, or in 10 patients in one treatment arm is summarized.

Source: ADaM.ADSL, ADaM.ADAE

Run Date: 2024-04-26T15:39:31

Data cutoff date of 31Mar2023

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Table GERMANY.3.1.3.2.1.8
Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term for Overall Danicopan Treatment
Up to Data Cut-off
Safety Set

System Organ Class (SOC) Preferred Term (PT)	D->D (N=57) n (%) (95% CI)	P->D (N=27) n (%) (95% CI)	Total (N=84) n (%) (95% CI)
Musculoskeletal and connective tissue disorders	22 (38.6) (26.0, 52.4)	6 (22.2) (8.6, 42.3)	28 (33.3) (23.4, 44.5)
Arthralgia	6 (10.5) (4.0, 21.5)	3 (11.1) (2.4, 29.2)	9 (10.7) (5.0, 19.4)
Back pain	4 (7.0) (1.9, 17.0)	3 (11.1) (2.4, 29.2)	7 (8.3) (3.4, 16.4)
Pain in extremity	7 (12.3) (5.1, 23.7)	1 (3.7) (0.1, 19.0)	8 (9.5) (4.2, 17.9)
Nervous system disorders	14 (24.6) (14.1, 37.8)	5 (18.5) (6.3, 38.1)	19 (22.6) (14.2, 33.0)
Headache	11 (19.3) (10.0, 31.9)	2 (7.4) (0.9, 24.3)	13 (15.5) (8.5, 25.0)
Psychiatric disorders	6 (10.5) (4.0, 21.5)	3 (11.1) (2.4, 29.2)	9 (10.7) (5.0, 19.4)

% = n/N*100, CI=Confidence Interval

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period are regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods is only counted once in the treatment period the event started.

SOCs and PTs are sorted alphabetically.

SOCs and PTs were coded using MedDRA version 25.1.

The 95% CI for each treatment group is computed by Clopper-Pearson method.

SOCs and PTs with at least 10% patients in a study arm, or in 10 patients in one treatment arm is summarized.

Source: ADaM.ADSL, ADaM.ADAE

Run Date: 2024-04-26T15:39:31

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Table GERMANY.3.1.3.2.1.8
Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term for Overall Danicopan Treatment
Up to Data Cut-off
Safety Set

System Organ Class (SOC) Preferred Term (PT)	D->D (N=57) n (%) (95% CI)	P->D (N=27) n (%) (95% CI)	Total (N=84) n (%) (95% CI)
Renal and urinary disorders	8 (14.0) (6.3, 25.8)	4 (14.8) (4.2, 33.7)	12 (14.3) (7.6, 23.6)
Reproductive system and breast disorders	7 (12.3) (5.1, 23.7)	3 (11.1) (2.4, 29.2)	10 (11.9) (5.9, 20.8)
Respiratory, thoracic and mediastinal disorders	19 (33.3) (21.4, 47.1)	6 (22.2) (8.6, 42.3)	25 (29.8) (20.3, 40.7)
Skin and subcutaneous tissue disorders	9 (15.8) (7.5, 27.9)	7 (25.9) (11.1, 46.3)	16 (19.0) (11.3, 29.1)
Vascular disorders	6 (10.5) (4.0, 21.5)	2 (7.4) (0.9, 24.3)	8 (9.5) (4.2, 17.9)

% = n/N*100, CI=Confidence Interval

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period are regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods is only counted once in the treatment period the event started.

SOCs and PTs are sorted alphabetically.

SOCs and PTs were coded using MedDRA version 25.1.

The 95% CI for each treatment group is computed by Clopper-Pearson method.

SOCs and PTs with at least 10% patients in a study arm, or in 10 patients in one treatment arm is summarized.

Source: ADaM.ADSL, ADaM.ADAE

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Table GERMANY.3.1.3.2.2.8
Severe Treatment-Emergent Adverse Events (CTCAE ≥ 3) by MedDRA System Organ Class and Preferred Term
for Overall Danicopan Treatment Up to Data Cut-off
Safety Set

System Organ Class (SOC) Preferred Term (PT)	D->D (N=57) n (%) (95% CI)	P->D (N=27) n (%) (95% CI)	Total (N=84) n (%) (95% CI)
Any Adverse Event	19 (33.3) (21.4, 47.1)	10 (37.0) (19.4, 57.6)	29 (34.5) (24.5, 45.7)
Blood and lymphatic system disorders	6 (10.5) (4.0, 21.5)	6 (22.2) (8.6, 42.3)	12 (14.3) (7.6, 23.6)
Anaemia	3 (5.3) (1.1, 14.6)	1 (3.7) (0.1, 19.0)	4 (4.8) (1.3, 11.7)
Haemolysis	0 (0.0, 6.3)	2 (7.4) (0.9, 24.3)	2 (2.4) (0.3, 8.3)
Neutropenia	3 (5.3) (1.1, 14.6)	1 (3.7) (0.1, 19.0)	4 (4.8) (1.3, 11.7)
Gastrointestinal disorders	3 (5.3) (1.1, 14.6)	1 (3.7) (0.1, 19.0)	4 (4.8) (1.3, 11.7)

% = n/N*100, CI=Confidence Interval

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

SOCs and PTs are sorted alphabetically.

SOCs and PTs were coded using MedDRA version 25.1.

The 95% CI for each treatment group is computed by Clopper-Pearson method.

SOCs and PTs with at least 5% patients in a study arm, or in 10 patients in one treatment arm is summarized.

Source: ADaM.ADSL, ADaM.ADAE

Run Date: 2024-04-26T15:39:32

Data cutoff date of 31Mar2023

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Table GERMANY.3.1.3.2.2.8
Severe Treatment-Emergent Adverse Events (CTCAE \geq 3) by MedDRA System Organ Class and Preferred Term
for Overall Danicopan Treatment Up to Data Cut-off
Safety Set

System Organ Class (SOC) Preferred Term (PT)	D->D (N=57) n (%) (95% CI)	P->D (N=27) n (%) (95% CI)	Total (N=84) n (%) (95% CI)
Infections and infestations	3 (5.3) (1.1, 14.6)	4 (14.8) (4.2, 33.7)	7 (8.3) (3.4, 16.4)
COVID-19	2 (3.5) (0.4, 12.1)	2 (7.4) (0.9, 24.3)	4 (4.8) (1.3, 11.7)
Injury, poisoning and procedural complications	1 (1.8) (0.0, 9.4)	2 (7.4) (0.9, 24.3)	3 (3.6) (0.7, 10.1)
Investigations	6 (10.5) (4.0, 21.5)	1 (3.7) (0.1, 19.0)	7 (8.3) (3.4, 16.4)
Alanine aminotransferase increased	3 (5.3) (1.1, 14.6)	0 (0.0, 12.8)	3 (3.6) (0.7, 10.1)

% = n/N*100, CI=Confidence Interval

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

SOCs and PTs are sorted alphabetically.

SOCs and PTs were coded using MedDRA version 25.1.

The 95% CI for each treatment group is computed by Clopper-Pearson method.

SOCs and PTs with at least 5% patients in a study arm, or in 10 patients in one treatment arm is summarized.

Source: ADaM.ADSL, ADaM.ADAE

Run Date: 2024-04-26T15:39:32

Data cutoff date of 31Mar2023

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Table GERMANY.3.1.3.2.3.8
Serious Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term for Overall Danicopan Treatment
Up to Data Cut-off
Safety Set

System Organ Class (SOC) Preferred Term (PT)	D->D (N=57) n (%) (95% CI)	P->D (N=27) n (%) (95% CI)	Total (N=84) n (%) (95% CI)
Any Adverse Event	9 (15.8) (7.5, 27.9)	8 (29.6) (13.8, 50.2)	17 (20.2) (12.3, 30.4)
Blood and lymphatic system disorders	0 (0.0, 6.3)	3 (11.1) (2.4, 29.2)	3 (3.6) (0.7, 10.1)
Haemolysis	0 (0.0, 6.3)	2 (7.4) (0.9, 24.3)	2 (2.4) (0.3, 8.3)
Infections and infestations	3 (5.3) (1.1, 14.6)	4 (14.8) (4.2, 33.7)	7 (8.3) (3.4, 16.4)
COVID-19	2 (3.5) (0.4, 12.1)	2 (7.4) (0.9, 24.3)	4 (4.8) (1.3, 11.7)

% = n/N*100, CI=Confidence Interval

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

SOCs and PTs are sorted alphabetically.

SOCs and PTs were coded using MedDRA version 25.1.

The 95% CI for each treatment group is computed by Clopper-Pearson method.

SOCs and PTs with at least 5% patients in a study arm, or in 10 patients in one treatment arm is summarized.

Source: ADaM.ADSL, ADaM.ADAE

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Table GERMANY.3.1.3.2.4.8
Treatment-Emergent Adverse Events Leading to Withdrawal of Study Drug by MedDRA System Organ Class and Preferred Term
for Overall Danicopan Treatment Up to Data Cut-off
Safety Set

System Organ Class (SOC) Preferred Term (PT)	D->D (N=57) n (%) (95% CI)	P->D (N=27) n (%) (95% CI)	Total (N=84) n (%) (95% CI)
Any Adverse Event	3 (5.3) (1.1, 14.6)	2 (7.4) (0.9, 24.3)	5 (6.0) (2.0, 13.3)
Gastrointestinal disorders	1 (1.8) (0.0, 9.4)	0 (0.0, 12.8)	1 (1.2) (0.0, 6.5)
Pancreatitis	1 (1.8) (0.0, 9.4)	0 (0.0, 12.8)	1 (1.2) (0.0, 6.5)
Hepatobiliary disorders	0 (0.0, 6.3)	2 (7.4) (0.9, 24.3)	2 (2.4) (0.3, 8.3)
Cholecystitis	0 (0.0, 6.3)	1 (3.7) (0.1, 19.0)	1 (1.2) (0.0, 6.5)
Hepatic function abnormal	0 (0.0, 6.3)	1 (3.7) (0.1, 19.0)	1 (1.2) (0.0, 6.5)

% = n/N*100, CI=Confidence Interval

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

SOCs and PTs are sorted alphabetically.

SOCs and PTs were coded using MedDRA version 25.1.

The 95% CI for each treatment group is computed by Clopper-Pearson method.

Source: ADaM.ADSL, ADaM.ADAE

Run Date: 2024-04-26T15:39:32

Data cutoff date of 31Mar2023

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Table GERMANY.3.1.3.2.4.8
Treatment-Emergent Adverse Events Leading to Withdrawal of Study Drug by MedDRA System Organ Class and Preferred Term
for Overall Danicopan Treatment Up to Data Cut-off
Safety Set

System Organ Class (SOC) Preferred Term (PT)	D->D (N=57) n (%) (95% CI)	P->D (N=27) n (%) (95% CI)	Total (N=84) n (%) (95% CI)
Investigations	3 (5.3) (1.1, 14.6)	0 (0.0, 12.8)	3 (3.6) (0.7, 10.1)
Alanine aminotransferase increased	1 (1.8) (0.0, 9.4)	0 (0.0, 12.8)	1 (1.2) (0.0, 6.5)
Aspartate aminotransferase increased	1 (1.8) (0.0, 9.4)	0 (0.0, 12.8)	1 (1.2) (0.0, 6.5)
Blood bilirubin increased	1 (1.8) (0.0, 9.4)	0 (0.0, 12.8)	1 (1.2) (0.0, 6.5)
Hepatic enzyme increased	1 (1.8) (0.0, 9.4)	0 (0.0, 12.8)	1 (1.2) (0.0, 6.5)

% = n/N*100, CI=Confidence Interval

In summarizing n(%), if a patient had multiple events for a particular SOC or PT, he/she is counted only once for that SOC or PT.

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

SOCs and PTs are sorted alphabetically.

SOCs and PTs were coded using MedDRA version 25.1.

The 95% CI for each treatment group is computed by Clopper-Pearson method.

Source: ADaM.ADSL, ADaM.ADAE

Run Date: 2024-04-26T15:39:32

Data cutoff date of 31Mar2023

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

3 Sicherheit

3.3 Unerwünschte Ereignisse von besonderem Interesse (AESI)

3.3.1 Unerwünschte Ereignisse von besonderem Interesse (AESI) innerhalb TP1

Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

3 Sicherheit

3.3 Unerwünschte Ereignisse von besonderem Interesse (AESI)

3.3.1 Unerwünschte Ereignisse von besonderem Interesse (AESI) innerhalb TP1

3.3.1.1 Subgruppenanalysen – Unerwünschte Ereignisse von besonderem Interesse (AESI) innerhalb TP1

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Table GERMANY.3.1.3.3.1
Overview of Treatment-Emergent Adverse Events of Special Interest (AESI) for Treatment Period 1
Safety Set

Meningococcal Infections

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value
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No data match the criteria for this report.

AESI = Adverse Events of Special Interest, % = $n/N \times 100$, RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.
TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.
Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal
AEs are coded using MedDRA version 25.1.
P-value is from the fisher exact test for treatment comparison. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.
Source: ADaM.ADSL, ADaM.ADAE
Run Date: 2024-04-26T15:36:08
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Table GERMANY.3.1.3.3.1
Overview of Treatment-Emergent Adverse Events of Special Interest (AESI) for Treatment Period 1
Safety Set

Liver Enzyme Elevations

	Danicopan (N=57) n (%) (95% CI)	Placebo (N=29) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value
Any AESI	8 (14.0) (6.3, 25.8)	3 (10.3) (2.2, 27.4)	1.36 (0.41, 7.61)	1.41 (0.30, 8.95)	0.04 (-0.15, 0.18)	0.7433
Serious AESI	1 (1.8) (0.0, 9.4)	0 (0.0, 11.9)	NA	NA	0.02 (-0.11, 0.10)	1.0000
Severe AESI	4 (7.0) (1.9, 17.0)	0 (0.0, 11.9)	NA	NA	0.07 (-0.06, 0.17)	0.2953
Non-severe AESI	6 (10.5) (4.0, 21.5)	3 (10.3) (2.2, 27.4)	1.02 (0.27, 7.56)	1.02 (0.20, 6.80)	0.00 (-0.18, 0.14)	1.0000

AESI = Adverse Events of Special Interest, % = n/N*100 , RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.
TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

P-value is from the fisher exact test for treatment comparison. The 95% CI for each treatment group is computed by Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

Source: ADaM.ADSL, ADaM.ADAE

Run Date: 2024-04-26T15:36:08

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

3 Sicherheit

3.3 Unerwünschte Ereignisse von besonderem Interesse (AESI)

3.3.2 Unerwünschte Ereignisse von besonderem Interesse (AESI) innerhalb der gesamten Behandlungsdauer (inkl. TP2 und LTE)

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Table GERMANY.3.1.3.3.8

Overview of Treatment-Emergent Adverse Events of Special Interest (AESI) for Overall Danicopan Treatment Up to Data Cut-off Safety Set

Meningococcal Infections

	D->D (N=57) n (%) (95% CI)	P->D (N=27) n (%) (95% CI)	Total (N=84) n (%) (95% CI)
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No data match the criteria for this report.

AESI = Adverse Events of Special Interest, % = n/N*100, CI=Confidence Interval

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

The 95% CI for each treatment group is computed by Clopper-Pearson method.

Source: ADaM.ADSL, ADaM.ADAE

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Table GERMANY.3.1.3.3.8

Overview of Treatment-Emergent Adverse Events of Special Interest (AESI) for Overall Danicopan Treatment Up to Data Cut-off Safety Set

Liver Enzyme Elevations

	D->D (N=57) n (%) (95% CI)	P->D (N=27) n (%) (95% CI)	Total (N=84) n (%) (95% CI)
Any AESI	10 (17.5) (8.7, 29.9)	4 (14.8) (4.2, 33.7)	14 (16.7) (9.4, 26.4)
Serious AESI	1 (1.8) (0.0, 9.4)	0 (0.0, 12.8)	1 (1.2) (0.0, 6.5)
Severe AESI	4 (7.0) (1.9, 17.0)	0 (0.0, 12.8)	4 (4.8) (1.3, 11.7)
Non-severe AESI	8 (14.0) (6.3, 25.8)	4 (14.8) (4.2, 33.7)	12 (14.3) (7.6, 23.6)

AESI = Adverse Events of Special Interest, % = n/N*100, CI=Confidence Interval

TEAE that has a starting date occurred during a certain Treatment Period is regarded as TEAE at that Treatment Period. Any TEAEs lasted across treatment periods are only counted once in the treatment period the event started.

Grade 1=mild; Grade 2=moderate; Grade 3=severe; Grade 4=life-threatening; Grade 5=Fatal

AEs are coded using MedDRA version 25.1.

The 95% CI for each treatment group is computed by Clopper-Pearson method.

Source: ADaM.ADSL, ADaM.ADAE

Run Date: 2024-04-26T15:46:22

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

3 Sicherheit

3.4 Hospitalisierungen anhand des Healthcare Resource Utilization (HRU)

3.4.1 Rücklaufquoten des HRU

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Table GERMANY.2.5.6.10
HRU Compliance Rate During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

How many times gone to an Emergency Room for treatment of PNH?

Visit	Statistics	D->D (N=57)	P->D (N=26)
Baseline	Expected Patients Per Visit	57	26
	Patients Per Visit	57	26
	Patients Who Completed Question, n (%)	50 (87.7)	22 (84.6)
Week 12	Expected Patients Per Visit	55	25
	Patients Per Visit	54	25
	Patients Who Completed Question, n (%)	50 (90.9)	23 (92.0)
Week 24	Expected Patients Per Visit	55	24
	Patients Per Visit	55	24
	Patients Who Completed Question, n (%)	44 (80.0)	22 (91.7)
Week 40	Expected Patients Per Visit	46	22
	Patients Per Visit	46	22
	Patients Who Completed Question, n (%)	38 (82.6)	21 (95.5)
Week 56	Expected Patients Per Visit	36	18
	Patients Per Visit	36	18
	Patients Who Completed Question, n (%)	28 (77.8)	16 (88.9)
Week 72	Expected Patients Per Visit	21	12
	Patients Per Visit	21	12
	Patients Who Completed Question, n (%)	16 (76.2)	10 (83.3)

Percentages are based on the total number of expected patients in each group.

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.6.10
HRU Compliance Rate During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

How many times gone to an Emergency Room for treatment of PNH?

Visit	Statistics	D->D (N=57)	P->D (N=26)
Week 88	Expected Patients Per Visit	14	8
	Patients Per Visit	14	8
	Patients Who Completed Question, n (%)	13 (92.9)	6 (75.0)
Week 104	Expected Patients Per Visit	4	2
	Patients Per Visit	4	2
	Patients Who Completed Question, n (%)	4 (100)	1 (50.0)

Percentages are based on the total number of expected patients in each group.

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

Run Date: 2024-04-26T15:50:50

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Table GERMANY.2.5.6.10
HRU Compliance Rate During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

How many times been admitted to a hospital for treatment of PNH?

Visit	Statistics	D->D (N=57)	P->D (N=26)
Baseline	Expected Patients Per Visit	57	26
	Patients Per Visit	57	26
	Patients Who Completed Question, n (%)	50 (87.7)	22 (84.6)
Week 12	Expected Patients Per Visit	55	25
	Patients Per Visit	54	25
	Patients Who Completed Question, n (%)	50 (90.9)	23 (92.0)
Week 24	Expected Patients Per Visit	55	24
	Patients Per Visit	55	24
	Patients Who Completed Question, n (%)	44 (80.0)	22 (91.7)
Week 40	Expected Patients Per Visit	46	22
	Patients Per Visit	46	22
	Patients Who Completed Question, n (%)	38 (82.6)	21 (95.5)
Week 56	Expected Patients Per Visit	36	18
	Patients Per Visit	36	18
	Patients Who Completed Question, n (%)	28 (77.8)	16 (88.9)
Week 72	Expected Patients Per Visit	21	12
	Patients Per Visit	21	12
	Patients Who Completed Question, n (%)	16 (76.2)	10 (83.3)

Percentages are based on the total number of expected patients in each group.

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.6.10
HRU Compliance Rate During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

How many times been admitted to a hospital for treatment of PNH?

Visit	Statistics	D->D (N=57)	P->D (N=26)
Week 88	Expected Patients Per Visit	14	8
	Patients Per Visit	14	8
	Patients Who Completed Question, n (%)	13 (92.9)	6 (75.0)
Week 104	Expected Patients Per Visit	4	2
	Patients Per Visit	4	2
	Patients Who Completed Question, n (%)	4 (100)	1 (50.0)

Percentages are based on the total number of expected patients in each group.

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Source: ADaM.ADSL, ADaM.ADQS

Data cutoff date of 31Mar2023

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3 Sicherheit

3.4 Hospitalisierungen anhand des Healthcare Resource Utilization (HRU)

3.4.2 Veränderung der Hospitalisierungen anhand des HRU im Vergleich zu Baseline

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Table GERMANY.2.5.6.1
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	50		22		72	
Mean (SD)	0.1 (0.27)		0.1 (0.29)		0.1 (0.28)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 1		0, 1	
Week 12						
n	50	46	23	20		
Mean (SD)	0.0 (0.00)	-0.1 (0.28)	0.0 (0.00)	-0.1 (0.31)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	-1, 0		
Week 24						
n	44	41	22	19		
Mean (SD)	0.0 (0.15)	-0.1 (0.26)	0.0 (0.00)	-0.1 (0.32)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 1	-1, 0	0, 0	-1, 0		

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Modified Randomized Set

Times Gone to an Emergency Room for PNH

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	38	37	21	18	59	55
Mean (SD)	0.0 (0.00)	-0.1 (0.23)	0.0 (0.00)	-0.1 (0.32)	0.0 (0.00)	-0.1 (0.26)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	-1, 0	0, 0	-1, 0
Week 56						
n	28	27	16	15	44	42
Mean (SD)	0.0 (0.19)	0.0 (0.34)	0.0 (0.00)	-0.1 (0.26)	0.0 (0.15)	0.0 (0.31)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 1	-1, 1	0, 0	-1, 0	0, 1	-1, 1
Week 72						
n	16	15	10	8	26	23
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Modified Randomized Set

Times Gone to an Emergency Room for PNH

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	13	12	6	5	19	17
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	4	4	1	1	5	5
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.1
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension
Modified Randomized Set

Times Admitted to a Hospital for PNH

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	50		22		72	
Mean (SD)	0.1 (0.39)		0.2 (0.53)		0.2 (0.43)	
Median	0.0		0.0		0.0	
Min, Max	0, 2		0, 2		0, 2	
Week 12						
n	50	46	23	20		
Mean (SD)	0.1 (0.42)	0.0 (0.59)	0.2 (0.60)	-0.1 (0.76)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 3	-2, 3	0, 2	-2, 2		
Week 24						
n	44	41	22	19		
Mean (SD)	0.0 (0.15)	-0.1 (0.30)	0.1 (0.29)	-0.2 (0.60)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 1	-1, 0	0, 1	-2, 1		

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Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	38	37	21	18	59	55
Mean (SD)	0.1 (0.32)	0.0 (0.50)	0.0 (0.00)	-0.3 (0.57)	0.0 (0.26)	-0.1 (0.53)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 2	-2, 2	0, 0	-2, 0	0, 2	-2, 2
Week 56						
n	28	27	16	15	44	42
Mean (SD)	0.0 (0.00)	0.0 (0.19)	0.2 (0.75)	-0.1 (0.80)	0.1 (0.45)	0.0 (0.49)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 3	-2, 2	0, 3	-2, 2
Week 72						
n	16	15	10	8	26	23
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.4 (0.74)	0.0 (0.00)	-0.1 (0.46)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-2, 0	0, 0	-2, 0

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Modified Randomized Set

Times Admitted to a Hospital for PNH

Visit/Statistics	D->D (N=57)		P->D (N=26)		Total (N=83)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	13	12	6	5	19	17
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.4 (0.89)	0.0 (0.00)	-0.1 (0.49)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-2, 0	0, 0	-2, 0
Week 104						
n	4	4	1	1	5	5
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

3 Sicherheit

3.4 Hospitalisierungen anhand des Healthcare Resource Utilization (HRU)

3.4.2 Veränderung der Hospitalisierungen anhand des Healthcare Resource Utilization (HRU) im Vergleich zu Baseline

3.4.2.1 Subgruppenanalysen – Veränderung der Hospitalisierungen anhand des HRU im Vergleich zu Baseline

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Table GERMANY.2.5.6.2
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		8		30	
Mean (SD)	0.1 (0.35)		0.3 (0.46)		0.2 (0.38)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 1		0, 1	
Week 12						
n	23	22	9	8		
Mean (SD)	0.0 (0.00)	-0.1 (0.35)	0.0 (0.00)	-0.3 (0.46)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	-1, 0		
Week 24						
n	20	20	9	8		
Mean (SD)	0.0 (0.00)	-0.2 (0.37)	0.0 (0.00)	-0.3 (0.46)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	-1, 0		

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	8	7	28	27
Mean (SD)	0.0 (0.00)	-0.1 (0.31)	0.0 (0.00)	-0.3 (0.49)	0.0 (0.00)	-0.1 (0.36)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	-1, 0	0, 0	-1, 0
Week 56						
n	15	15	5	4	20	19
Mean (SD)	0.0 (0.00)	-0.1 (0.35)	0.0 (0.00)	-0.3 (0.50)	0.0 (0.00)	-0.2 (0.37)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	-1, 0	0, 0	-1, 0
Week 72						
n	7	7	1	1	8	8
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	1	1	7	7
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.0 (NA)	0.0 (NA)			0.0 (NA)	0.0 (NA)
Median	0.0	0.0			0.0	0.0
Min, Max	0, 0	0, 0			0, 0	0, 0

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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Times Admitted to a Hospital for PNH

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		8		30	
Mean (SD)	0.2 (0.50)		0.3 (0.46)		0.2 (0.48)	
Median	0.0		0.0		0.0	
Min, Max	0, 2		0, 1		0, 2	
Week 12						
n	23	22	9	8		
Mean (SD)	0.1 (0.63)	0.0 (0.84)	0.0 (0.00)	-0.3 (0.46)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 3	-2, 3	0, 0	-1, 0		
Week 24						
n	20	20	9	8		
Mean (SD)	0.1 (0.22)	-0.2 (0.37)	0.0 (0.00)	-0.3 (0.46)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 1	-1, 0	0, 0	-1, 0		

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This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.6.2
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Times Admitted to a Hospital for PNH

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	20	20	8	7	28	27
Mean (SD)	0.1 (0.45)	-0.1 (0.69)	0.0 (0.00)	-0.3 (0.49)	0.1 (0.38)	-0.1 (0.64)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 2	-2, 2	0, 0	-1, 0	0, 2	-2, 2
Week 56						
n	15	15	5	4	20	19
Mean (SD)	0.0 (0.00)	-0.1 (0.26)	0.0 (0.00)	-0.3 (0.50)	0.0 (0.00)	-0.1 (0.32)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	-1, 0	0, 0	-1, 0
Week 72						
n	7	7	1	1	8	8
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.2
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Times Admitted to a Hospital for PNH

Male

Visit/Statistics	D->D (N=23)		P->D (N=9)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	1	1	7	7
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.0 (NA)	0.0 (NA)			0.0 (NA)	0.0 (NA)
Median	0.0	0.0			0.0	0.0
Min, Max	0, 0	0, 0			0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.2
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		14		42	
Mean (SD)	0.0 (0.19)		0.0 (0.00)		0.0 (0.15)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 0		0, 1	
Week 12						
n	27	24	14	12		
Mean (SD)	0.0 (0.00)	0.0 (0.20)	0.0 (0.00)	0.0 (0.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	0, 0		
Week 24						
n	24	21	13	11		
Mean (SD)	0.0 (0.20)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 1	0, 0	0, 0	0, 0		

HRU is Resource Utilization Patient Questionnaire.

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Table GERMANY.2.5.6.2
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	17	13	11	31	28
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 56						
n	13	12	11	11	24	23
Mean (SD)	0.1 (0.28)	0.1 (0.29)	0.0 (0.00)	0.0 (0.00)	0.0 (0.20)	0.0 (0.21)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 1	0, 1	0, 0	0, 0	0, 1	0, 1
Week 72						
n	9	8	9	7	18	15
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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Table GERMANY.2.5.6.2
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	6	5	4	12	10
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	3	3	1	1	4	4
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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Table GERMANY.2.5.6.2
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Times Admitted to a Hospital for PNH

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		14		42	
Mean (SD)	0.1 (0.26)		0.2 (0.58)		0.1 (0.40)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 2		0, 2	
Week 12						
n	27	24	14	12		
Mean (SD)	0.0 (0.00)	0.0 (0.20)	0.4 (0.74)	0.1 (0.90)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 2	-2, 2		
Week 24						
n	24	21	13	11		
Mean (SD)	0.0 (0.00)	0.0 (0.22)	0.2 (0.38)	-0.1 (0.70)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 1	-2, 1		

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Table GERMANY.2.5.6.2
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During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Times Admitted to a Hospital for PNH

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	18	17	13	11	31	28
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.3 (0.65)	0.0 (0.00)	-0.1 (0.42)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-2, 0	0, 0	-2, 0
Week 56						
n	13	12	11	11	24	23
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.3 (0.90)	0.0 (0.89)	0.1 (0.61)	0.0 (0.60)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 3	-2, 2	0, 3	-2, 2
Week 72						
n	9	8	9	7	18	15
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.4 (0.79)	0.0 (0.00)	-0.2 (0.56)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-2, 0	0, 0	-2, 0

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Table GERMANY.2.5.6.2
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Sex
Modified Randomized Set

Times Admitted to a Hospital for PNH

Female

Visit/Statistics	D->D (N=34)		P->D (N=17)		Total (N=51)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	6	5	4	12	10
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.5 (1.00)	0.0 (0.00)	-0.2 (0.63)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-2, 0	0, 0	-2, 0
Week 104						
n	3	3	1	1	4	4
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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Table GERMANY.2.5.6.3
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		17		51	
Mean (SD)	0.1 (0.29)		0.1 (0.33)		0.1 (0.30)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 1		0, 1	
Week 12						
n	37	33	17	15		
Mean (SD)	0.0 (0.00)	-0.1 (0.29)	0.0 (0.00)	-0.1 (0.35)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	-1, 0		
Week 24						
n	32	29	16	14		
Mean (SD)	0.0 (0.18)	-0.1 (0.26)	0.0 (0.00)	-0.1 (0.36)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 1	-1, 0	0, 0	-1, 0		

HRU is Resource Utilization Patient Questionnaire.

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Table GERMANY.2.5.6.3
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	26	16	14	43	40
Mean (SD)	0.0 (0.00)	0.0 (0.20)	0.0 (0.00)	-0.1 (0.36)	0.0 (0.00)	-0.1 (0.27)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	-1, 0	0, 0	-1, 0
Week 56						
n	19	18	13	13	32	31
Mean (SD)	0.1 (0.23)	0.0 (0.34)	0.0 (0.00)	-0.1 (0.28)	0.0 (0.18)	0.0 (0.31)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 1	-1, 1	0, 0	-1, 0	0, 1	-1, 1
Week 72						
n	11	10	9	7	20	17
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.3

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	8	7	5	4	13	11
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	3	3	1	1	4	4
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

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Table GERMANY.2.5.6.3
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Times Admitted to a Hospital for PNH

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		17		51	
Mean (SD)	0.1 (0.41)		0.2 (0.39)		0.1 (0.40)	
Median	0.0		0.0		0.0	
Min, Max	0, 2		0, 1		0, 2	
Week 12						
n	37	33	17	15		
Mean (SD)	0.1 (0.49)	0.0 (0.68)	0.3 (0.69)	0.1 (0.70)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 3	-2, 3	0, 2	-1, 2		
Week 24						
n	32	29	16	14		
Mean (SD)	0.0 (0.18)	-0.1 (0.31)	0.1 (0.34)	-0.1 (0.47)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 1	-1, 0	0, 1	-1, 1		

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This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.6.3

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Times Admitted to a Hospital for PNH

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	27	26	16	14	43	40
Mean (SD)	0.1 (0.38)	0.0 (0.57)	0.0 (0.00)	-0.2 (0.43)	0.0 (0.30)	-0.1 (0.53)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 2	-2, 2	0, 0	-1, 0	0, 2	-2, 2
Week 56						
n	19	18	13	13	32	31
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.2 (0.83)	0.1 (0.64)	0.1 (0.53)	0.0 (0.41)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 3	-1, 2	0, 3	-1, 2
Week 72						
n	11	10	9	7	20	17
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.1 (0.38)	0.0 (0.00)	-0.1 (0.24)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-1, 0	0, 0	-1, 0

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Table GERMANY.2.5.6.3

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Times Admitted to a Hospital for PNH

< 65 Years

Visit/Statistics	D->D (N=41)		P->D (N=20)		Total (N=61)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	8	7	5	4	13	11
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	3	3	1	1	4	4
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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Table GERMANY.2.5.6.3
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		5		21	
Mean (SD)	0.1 (0.25)		0.0 (0.00)		0.0 (0.22)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 0		0, 1	
Week 12						
n	13	13	6	5		
Mean (SD)	0.0 (0.00)	-0.1 (0.28)	0.0 (0.00)	0.0 (0.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	0, 0		
Week 24						
n	12	12	6	5		
Mean (SD)	0.0 (0.00)	-0.1 (0.29)	0.0 (0.00)	0.0 (0.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	0, 0		

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.3
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	11	11	5	4	16	15
Mean (SD)	0.0 (0.00)	-0.1 (0.30)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.1 (0.26)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	0, 0	0, 0	-1, 0
Week 56						
n	9	9	3	2	12	11
Mean (SD)	0.0 (0.00)	-0.1 (0.33)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.1 (0.30)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	0, 0	0, 0	-1, 0
Week 72						
n	5	5	1	1	6	6
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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Table GERMANY.2.5.6.3
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	5	5	1	1	6	6
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.0 (NA)	0.0 (NA)			0.0 (NA)	0.0 (NA)
Median	0.0	0.0			0.0	0.0
Min, Max	0, 0	0, 0			0, 0	0, 0

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Table GERMANY.2.5.6.3
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Age at Informed Consent (< 65 and ≥ 65 Years)
Modified Randomized Set

Times Admitted to a Hospital for PNH

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		5		21	
Mean (SD)	0.1 (0.34)		0.4 (0.89)		0.2 (0.51)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 2		0, 2	
Week 12						
n	13	13	6	5		
Mean (SD)	0.0 (0.00)	-0.1 (0.28)	0.0 (0.00)	-0.4 (0.89)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	-2, 0		
Week 24						
n	12	12	6	5		
Mean (SD)	0.0 (0.00)	-0.1 (0.29)	0.0 (0.00)	-0.4 (0.89)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	-2, 0		

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Table GERMANY.2.5.6.3

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Times Admitted to a Hospital for PNH

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	11	11	5	4	16	15
Mean (SD)	0.0 (0.00)	-0.1 (0.30)	0.0 (0.00)	-0.5 (1.00)	0.0 (0.00)	-0.2 (0.56)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	-2, 0	0, 0	-2, 0
Week 56						
n	9	9	3	2	12	11
Mean (SD)	0.0 (0.00)	-0.1 (0.33)	0.0 (0.00)	-1.0 (1.41)	0.0 (0.00)	-0.3 (0.65)
Median	0.0	0.0	0.0	-1.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	-2, 0	0, 0	-2, 0
Week 72						
n	5	5	1	1	6	6
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	-2.0 (NA)	0.0 (0.00)	-0.3 (0.82)
Median	0.0	0.0	0.0	-2.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-2, -2	0, 0	-2, 0

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This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.6.3

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by Age at Informed Consent (< 65 and ≥ 65 Years) Modified Randomized Set

Times Admitted to a Hospital for PNH

≥ 65 Years

Visit/Statistics	D->D (N=16)		P->D (N=6)		Total (N=22)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	5	5	1	1	6	6
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	-2.0 (NA)	0.0 (0.00)	-0.3 (0.82)
Median	0.0	0.0	0.0	-2.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-2, -2	0, 0	-2, 0
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.0 (NA)	0.0 (NA)			0.0 (NA)	0.0 (NA)
Median	0.0	0.0			0.0	0.0
Min, Max	0, 0	0, 0			0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.4
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		11		32	
Mean (SD)	0.1 (0.36)		0.2 (0.40)		0.2 (0.37)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 1		0, 1	
Week 12						
n	19	17	11	9		
Mean (SD)	0.0 (0.00)	-0.2 (0.39)	0.0 (0.00)	-0.2 (0.44)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	-1, 0		
Week 24						
n	17	15	11	9		
Mean (SD)	0.1 (0.24)	-0.1 (0.35)	0.0 (0.00)	-0.2 (0.44)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 1	-1, 0	0, 0	-1, 0		

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Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.6.4
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	15	14	12	10	27	24
Mean (SD)	0.0 (0.00)	-0.1 (0.27)	0.0 (0.00)	-0.2 (0.42)	0.0 (0.00)	-0.1 (0.34)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	-1, 0	0, 0	-1, 0
Week 56						
n	9	9	10	10	19	19
Mean (SD)	0.0 (0.00)	-0.1 (0.33)	0.0 (0.00)	-0.1 (0.32)	0.0 (0.00)	-0.1 (0.32)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	-1, 0	0, 0	-1, 0
Week 72						
n	7	6	7	5	14	11
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.4
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	5	4	3	10	8
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.0 (NA)	0.0 (NA)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.4
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Times Admitted to a Hospital for PNH

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	21		11		32	
Mean (SD)	0.3 (0.56)		0.5 (0.69)		0.3 (0.60)	
Median	0.0		0.0		0.0	
Min, Max	0, 2		0, 2		0, 2	
Week 12						
n	19	17	11	9		
Mean (SD)	0.2 (0.69)	-0.1 (0.99)	0.5 (0.82)	-0.1 (1.17)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 3	-2, 3	0, 2	-2, 2		
Week 24						
n	17	15	11	9		
Mean (SD)	0.1 (0.24)	-0.3 (0.46)	0.1 (0.30)	-0.4 (0.73)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 1	-1, 0	0, 1	-2, 0		

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Table GERMANY.2.5.6.4
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Times Admitted to a Hospital for PNH

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	15	14	12	10	27	24
Mean (SD)	0.1 (0.52)	-0.1 (0.83)	0.0 (0.00)	-0.5 (0.71)	0.1 (0.38)	-0.3 (0.79)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 2	-2, 2	0, 0	-2, 0	0, 2	-2, 2
Week 56						
n	9	9	10	10	19	19
Mean (SD)	0.0 (0.00)	-0.1 (0.33)	0.3 (0.95)	-0.1 (0.99)	0.2 (0.69)	-0.1 (0.74)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 3	-2, 2	0, 3	-2, 2
Week 72						
n	7	6	7	5	14	11
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.6 (0.89)	0.0 (0.00)	-0.3 (0.65)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-2, 0	0, 0	-2, 0

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Table GERMANY.2.5.6.4
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Times Admitted to a Hospital for PNH

Europe

Visit/Statistics	D->D (N=25)		P->D (N=13)		Total (N=38)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	5	4	3	10	8
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.7 (1.15)	0.0 (0.00)	-0.3 (0.71)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-2, 0	0, 0	-2, 0
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.0 (NA)	0.0 (NA)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.4
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	29		11		40	
Mean (SD)	0.0 (0.19)		0.0 (0.00)		0.0 (0.16)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 0		0, 1	
Week 12						
n	31	29	12	11		
Mean (SD)	0.0 (0.00)	0.0 (0.19)	0.0 (0.00)	0.0 (0.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	0, 0		
Week 24						
n	27	26	11	10		
Mean (SD)	0.0 (0.00)	0.0 (0.20)	0.0 (0.00)	0.0 (0.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	0, 0		

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.4
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	23	23	9	8	32	31
Mean (SD)	0.0 (0.00)	0.0 (0.21)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.18)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	0, 0	0, 0	-1, 0
Week 56						
n	19	18	6	5	25	23
Mean (SD)	0.1 (0.23)	0.0 (0.34)	0.0 (0.00)	0.0 (0.00)	0.0 (0.20)	0.0 (0.30)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 1	-1, 1	0, 0	0, 0	0, 1	-1, 1
Week 72						
n	9	9	3	3	12	12
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.4
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	7	2	2	9	9
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	3	3	0	0	3	3
Mean (SD)	0.0 (0.00)	0.0 (0.00)			0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0			0.0	0.0
Min, Max	0, 0	0, 0			0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.6.4
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Times Admitted to a Hospital for PNH

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	29		11		40	
Mean (SD)	0.0 (0.00)		0.0 (0.00)		0.0 (0.00)	
Median	0.0		0.0		0.0	
Min, Max	0, 0		0, 0		0, 0	
Week 12						
n	31	29	12	11		
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	0, 0	0, 0	0, 0		
Week 24						
n	27	26	11	10		
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.1 (0.30)	0.1 (0.32)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	0, 0	0, 1	0, 1		

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.6.4
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Times Admitted to a Hospital for PNH

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	23	23	9	8	32	31
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 56						
n	19	18	6	5	25	23
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 72						
n	9	9	3	3	12	12
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.4
Summary of Actual Values and Change from Baseline in HRU
During Treatment Period 1 and Period 2 and Long Term Extension by Region
Modified Randomized Set

Times Admitted to a Hospital for PNH

Rest of World

Visit/Statistics	D->D (N=32)		P->D (N=13)		Total (N=45)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	7	2	2	9	9
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	3	3	0	0	3	3
Mean (SD)	0.0 (0.00)	0.0 (0.00)			0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0			0.0	0.0
Min, Max	0, 0	0, 0			0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.5
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions
During 6 Months Prior to Screening)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	0.0 (0.21)		0.1 (0.33)		0.1 (0.25)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 1		0, 1	
Week 12						
n	21	21	10	9		
Mean (SD)	0.0 (0.00)	0.0 (0.22)	0.0 (0.00)	-0.1 (0.33)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	-1, 0		
Week 24						
n	19	18	8	7		
Mean (SD)	0.0 (0.00)	-0.1 (0.24)	0.0 (0.00)	-0.1 (0.38)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	-1, 0		

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.6.5

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Times Gone to an Emergency Room for PNH

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	16	8	7	25	23
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.1 (0.38)	0.0 (0.00)	0.0 (0.21)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-1, 0	0, 0	-1, 0
Week 56						
n	10	10	7	6	17	16
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 72						
n	6	6	5	5	11	11
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

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Table GERMANY.2.5.6.5

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Times Gone to an Emergency Room for PNH

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	5	1	1	7	6
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	3	3	0	0	3	3
Mean (SD)	0.0 (0.00)	0.0 (0.00)			0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0			0.0	0.0
Min, Max	0, 0	0, 0			0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

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Table GERMANY.2.5.6.5
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions
During 6 Months Prior to Screening)
Modified Randomized Set

Times Admitted to a Hospital for PNH

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		9		31	
Mean (SD)	0.1 (0.29)		0.2 (0.44)		0.1 (0.34)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 1		0, 1	
Week 12						
n	21	21	10	9		
Mean (SD)	0.1 (0.65)	0.1 (0.70)	0.4 (0.84)	0.2 (0.83)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 3	-1, 3	0, 2	-1, 2		
Week 24						
n	19	18	8	7		
Mean (SD)	0.0 (0.00)	-0.1 (0.24)	0.3 (0.46)	0.0 (0.58)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 1	-1, 1		

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.5
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions
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Modified Randomized Set

Times Admitted to a Hospital for PNH

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	17	16	8	7	25	23
Mean (SD)	0.1 (0.49)	0.1 (0.50)	0.0 (0.00)	-0.3 (0.49)	0.1 (0.40)	0.0 (0.52)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 2	0, 2	0, 0	-1, 0	0, 2	-1, 2
Week 56						
n	10	10	7	6	17	16
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.4 (1.13)	0.3 (0.82)	0.2 (0.73)	0.1 (0.50)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 3	0, 2	0, 3	0, 2
Week 72						
n	6	6	5	5	11	11
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.2 (0.45)	0.0 (0.00)	-0.1 (0.30)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-1, 0	0, 0	-1, 0

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Table GERMANY.2.5.6.5

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions During 6 Months Prior to Screening) Modified Randomized Set

Times Admitted to a Hospital for PNH

> 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=24)		P->D (N=10)		Total (N=34)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	5	1	1	7	6
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	3	3	0	0	3	3
Mean (SD)	0.0 (0.00)	0.0 (0.00)			0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0			0.0	0.0
Min, Max	0, 0	0, 0			0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:49:58

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.6.5
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions
During 6 Months Prior to Screening)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		13		41	
Mean (SD)	0.1 (0.31)		0.1 (0.28)		0.1 (0.30)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 1		0, 1	
Week 12						
n	29	25	13	11		
Mean (SD)	0.0 (0.00)	-0.1 (0.33)	0.0 (0.00)	-0.1 (0.30)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	-1, 0		
Week 24						
n	25	23	14	12		
Mean (SD)	0.0 (0.20)	-0.1 (0.29)	0.0 (0.00)	-0.1 (0.29)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 1	-1, 0	0, 0	-1, 0		

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.6.5
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions
During 6 Months Prior to Screening)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	13	11	34	32
Mean (SD)	0.0 (0.00)	-0.1 (0.30)	0.0 (0.00)	-0.1 (0.30)	0.0 (0.00)	-0.1 (0.30)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	-1, 0	0, 0	-1, 0
Week 56						
n	18	17	9	9	27	26
Mean (SD)	0.1 (0.24)	-0.1 (0.43)	0.0 (0.00)	-0.1 (0.33)	0.0 (0.19)	-0.1 (0.39)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 1	-1, 1	0, 0	-1, 0	0, 1	-1, 1
Week 72						
n	10	9	5	3	15	12
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions
During 6 Months Prior to Screening)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	7	5	4	12	11
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.0 (NA)	0.0 (NA)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.5
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions
During 6 Months Prior to Screening)
Modified Randomized Set

Times Admitted to a Hospital for PNH

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	28		13		41	
Mean (SD)	0.1 (0.45)		0.2 (0.60)		0.2 (0.50)	
Median	0.0		0.0		0.0	
Min, Max	0, 2		0, 2		0, 2	
Week 12						
n	29	25	13	11		
Mean (SD)	0.0 (0.00)	-0.2 (0.47)	0.1 (0.28)	-0.3 (0.65)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-2, 0	0, 1	-2, 0		
Week 24						
n	25	23	14	12		
Mean (SD)	0.0 (0.20)	-0.1 (0.34)	0.0 (0.00)	-0.3 (0.62)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 1	-1, 0	0, 0	-2, 0		

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This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Modified Randomized Set

Times Admitted to a Hospital for PNH

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	21	21	13	11	34	32
Mean (SD)	0.0 (0.00)	-0.1 (0.48)	0.0 (0.00)	-0.3 (0.65)	0.0 (0.00)	-0.2 (0.54)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-2, 0	0, 0	-2, 0	0, 0	-2, 0
Week 56						
n	18	17	9	9	27	26
Mean (SD)	0.0 (0.00)	-0.1 (0.24)	0.0 (0.00)	-0.3 (0.71)	0.0 (0.00)	-0.2 (0.46)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	-2, 0	0, 0	-2, 0
Week 72						
n	10	9	5	3	15	12
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.7 (1.15)	0.0 (0.00)	-0.2 (0.58)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-2, 0	0, 0	-2, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.5
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by the Randomization Stratification Factors of Transfusion History (> 2 or ≤ 2 Transfusions
During 6 Months Prior to Screening)
Modified Randomized Set

Times Admitted to a Hospital for PNH

≤ 2 Transfusions During 6 Months Prior to Screening

Visit/Statistics	D->D (N=33)		P->D (N=16)		Total (N=49)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	7	5	4	12	11
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.5 (1.00)	0.0 (0.00)	-0.2 (0.60)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-2, 0	0, 0	-2, 0
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.0 (NA)	0.0 (NA)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.6

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Times Gone to an Emergency Room for PNH

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	30		14		44	
Mean (SD)	0.1 (0.31)		0.1 (0.27)		0.1 (0.29)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 1		0, 1	
Week 12						
n	30	27	15	14		
Mean (SD)	0.0 (0.00)	-0.1 (0.32)	0.0 (0.00)	-0.1 (0.27)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	-1, 0		
Week 24						
n	29	27	12	11		
Mean (SD)	0.0 (0.00)	-0.1 (0.32)	0.0 (0.00)	-0.1 (0.30)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	-1, 0		

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Table GERMANY.2.5.6.6
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	24	23	11	10	35	33
Mean (SD)	0.0 (0.00)	-0.1 (0.29)	0.0 (0.00)	-0.1 (0.32)	0.0 (0.00)	-0.1 (0.29)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	-1, 0	0, 0	-1, 0
Week 56						
n	17	17	10	10	27	27
Mean (SD)	0.1 (0.24)	-0.1 (0.43)	0.0 (0.00)	0.0 (0.00)	0.0 (0.19)	0.0 (0.34)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 1	-1, 1	0, 0	0, 0	0, 1	-1, 1
Week 72						
n	9	8	7	6	16	14
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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Table GERMANY.2.5.6.6

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Times Gone to an Emergency Room for PNH

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	6	4	3	11	9
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	2	2	0	0	2	2
Mean (SD)	0.0 (0.00)	0.0 (0.00)			0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0			0.0	0.0
Min, Max	0, 0	0, 0			0, 0	0, 0

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This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.6

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Times Admitted to a Hospital for PNH

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	30		14		44	
Mean (SD)	0.1 (0.31)		0.2 (0.58)		0.1 (0.41)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 2		0, 2	
Week 12						
n	30	27	15	14		
Mean (SD)	0.1 (0.55)	0.0 (0.65)	0.1 (0.52)	-0.1 (0.83)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 3	-1, 3	0, 2	-2, 2		
Week 24						
n	29	27	12	11		
Mean (SD)	0.0 (0.00)	-0.1 (0.27)	0.1 (0.29)	-0.2 (0.75)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 1	-2, 1		

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.6.6

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Times Admitted to a Hospital for PNH

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	24	23	11	10	35	33
Mean (SD)	0.1 (0.41)	0.0 (0.47)	0.0 (0.00)	-0.3 (0.67)	0.1 (0.34)	-0.1 (0.56)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 2	-1, 2	0, 0	-2, 0	0, 2	-2, 2
Week 56						
n	17	17	10	10	27	27
Mean (SD)	0.0 (0.00)	-0.1 (0.24)	0.0 (0.00)	-0.2 (0.63)	0.0 (0.00)	-0.1 (0.42)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	-2, 0	0, 0	-2, 0
Week 72						
n	9	8	7	6	16	14
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.3 (0.82)	0.0 (0.00)	-0.1 (0.53)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-2, 0	0, 0	-2, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.6.6

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Times Admitted to a Hospital for PNH

Screening Hgb Level < 8.5 g/dL

Visit/Statistics	D->D (N=35)		P->D (N=15)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	7	6	4	3	11	9
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.7 (1.15)	0.0 (0.00)	-0.2 (0.67)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-2, 0	0, 0	-2, 0
Week 104						
n	2	2	0	0	2	2
Mean (SD)	0.0 (0.00)	0.0 (0.00)			0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0			0.0	0.0
Min, Max	0, 0	0, 0			0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Table GERMANY.2.5.6.6

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	20		8		28	
Mean (SD)	0.1 (0.22)		0.1 (0.35)		0.1 (0.26)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 1		0, 1	
Week 12						
n	20	19	8	6		
Mean (SD)	0.0 (0.00)	-0.1 (0.23)	0.0 (0.00)	-0.2 (0.41)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	-1, 0		
Week 24						
n	15	14	10	8		
Mean (SD)	0.1 (0.26)	0.0 (0.00)	0.0 (0.00)	-0.1 (0.35)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 1	0, 0	0, 0	-1, 0		

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.6.6
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	14	14	10	8	24	22
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.1 (0.35)	0.0 (0.00)	0.0 (0.21)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-1, 0	0, 0	-1, 0
Week 56						
n	11	10	6	5	17	15
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.2 (0.45)	0.0 (0.00)	-0.1 (0.26)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-1, 0	0, 0	-1, 0
Week 72						
n	7	7	3	2	10	9
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.6.6

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Times Gone to an Emergency Room for PNH

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	2	2	8	8
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	2	2	1	1	3	3
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.6.6

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Times Admitted to a Hospital for PNH

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	20		8		28	
Mean (SD)	0.2 (0.49)		0.3 (0.46)		0.2 (0.48)	
Median	0.0		0.0		0.0	
Min, Max	0, 2		0, 1		0, 2	
Week 12						
n	20	19	8	6		
Mean (SD)	0.0 (0.00)	-0.2 (0.50)	0.4 (0.74)	0.0 (0.63)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-2, 0	0, 2	-1, 1		
Week 24						
n	15	14	10	8		
Mean (SD)	0.1 (0.26)	-0.1 (0.36)	0.1 (0.32)	-0.1 (0.35)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 1	-1, 0	0, 1	-1, 0		

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.6.6

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Times Admitted to a Hospital for PNH

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	14	14	10	8	24	22
Mean (SD)	0.0 (0.00)	-0.1 (0.53)	0.0 (0.00)	-0.3 (0.46)	0.0 (0.00)	-0.2 (0.50)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-2, 0	0, 0	-1, 0	0, 0	-2, 0
Week 56						
n	11	10	6	5	17	15
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.5 (1.22)	0.2 (1.10)	0.2 (0.73)	0.1 (0.59)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 3	-1, 2	0, 3	-1, 2
Week 72						
n	7	7	3	2	10	9
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.5 (0.71)	0.0 (0.00)	-0.1 (0.33)
Median	0.0	0.0	0.0	-0.5	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-1, 0	0, 0	-1, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.6.6

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Screening Hgb Level (< 8.5 and ≥ 8.5 g/dL) Modified Randomized Set

Times Admitted to a Hospital for PNH

Screening Hgb Level ≥ 8.5 g/dL

Visit/Statistics	D->D (N=22)		P->D (N=11)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	2	2	8	8
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	2	2	1	1	3	3
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.6.7
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		8		24	
Mean (SD)	0.1 (0.25)		0.0 (0.00)		0.0 (0.20)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 0		0, 1	
Week 12						
n	16	14	10	7		
Mean (SD)	0.0 (0.00)	-0.1 (0.27)	0.0 (0.00)	0.0 (0.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	0, 0		
Week 24						
n	13	11	9	6		
Mean (SD)	0.0 (0.00)	-0.1 (0.30)	0.0 (0.00)	0.0 (0.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	0, 0		

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:49:58

Data cutoff date of 31Mar2023

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Table GERMANY.2.5.6.7
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	14	13	10	7	24	20
Mean (SD)	0.0 (0.00)	-0.1 (0.28)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.1 (0.22)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	0, 0	0, 0	-1, 0
Week 56						
n	9	8	9	8	18	16
Mean (SD)	0.0 (0.00)	-0.1 (0.35)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.1 (0.25)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	0, 0	0, 0	-1, 0
Week 72						
n	6	5	7	5	13	10
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.6.7
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	5	4	3	2	8	6
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.0 (NA)	0.0 (NA)			0.0 (NA)	0.0 (NA)
Median	0.0	0.0			0.0	0.0
Min, Max	0, 0	0, 0			0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.6.7
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Times Admitted to a Hospital for PNH

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	16		8		24	
Mean (SD)	0.2 (0.54)		0.4 (0.74)		0.3 (0.61)	
Median	0.0		0.0		0.0	
Min, Max	0, 2		0, 2		0, 2	
Week 12						
n	16	14	10	7		
Mean (SD)	0.2 (0.75)	0.0 (1.04)	0.5 (0.85)	0.1 (1.21)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 3	-2, 3	0, 2	-2, 2		
Week 24						
n	13	11	9	6		
Mean (SD)	0.1 (0.28)	-0.2 (0.40)	0.1 (0.33)	-0.3 (0.82)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 1	-1, 0	0, 1	-2, 0		

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.7
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by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Times Admitted to a Hospital for PNH

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	14	13	10	7	24	20
Mean (SD)	0.1 (0.53)	-0.1 (0.86)	0.0 (0.00)	-0.4 (0.79)	0.1 (0.41)	-0.2 (0.83)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 2	-2, 2	0, 0	-2, 0	0, 2	-2, 2
Week 56						
n	9	8	9	8	18	16
Mean (SD)	0.0 (0.00)	-0.1 (0.35)	0.3 (1.00)	0.0 (1.07)	0.2 (0.71)	-0.1 (0.77)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 3	-2, 2	0, 3	-2, 2
Week 72						
n	6	5	7	5	13	10
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.6 (0.89)	0.0 (0.00)	-0.3 (0.67)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-2, 0	0, 0	-2, 0

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Table GERMANY.2.5.6.7
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Times Admitted to a Hospital for PNH

Background C5 Inhibitor Therapy Eculizumab

Visit/Statistics	D->D (N=21)		P->D (N=12)		Total (N=33)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	5	4	3	2	8	6
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-1.0 (1.41)	0.0 (0.00)	-0.3 (0.82)
Median	0.0	0.0	0.0	-1.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-2, 0	0, 0	-2, 0
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.0 (NA)	0.0 (NA)			0.0 (NA)	0.0 (NA)
Median	0.0	0.0			0.0	0.0
Min, Max	0, 0	0, 0			0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

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Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

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Table GERMANY.2.5.6.7
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		14		48	
Mean (SD)	0.1 (0.29)		0.1 (0.36)		0.1 (0.31)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 1		0, 1	
Week 12						
n	34	32	13	13		
Mean (SD)	0.0 (0.00)	-0.1 (0.30)	0.0 (0.00)	-0.2 (0.38)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	-1, 0		
Week 24						
n	31	30	13	13		
Mean (SD)	0.0 (0.18)	-0.1 (0.25)	0.0 (0.00)	-0.2 (0.38)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 1	-1, 0	0, 0	-1, 0		

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

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Table GERMANY.2.5.6.7
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	24	24	11	11	35	35
Mean (SD)	0.0 (0.00)	0.0 (0.20)	0.0 (0.00)	-0.2 (0.40)	0.0 (0.00)	-0.1 (0.28)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	-1, 0	0, 0	-1, 0
Week 56						
n	19	19	7	7	26	26
Mean (SD)	0.1 (0.23)	0.0 (0.33)	0.0 (0.00)	-0.1 (0.38)	0.0 (0.20)	0.0 (0.34)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 1	-1, 1	0, 0	-1, 0	0, 1	-1, 1
Week 72						
n	10	10	3	3	13	13
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.7
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	8	8	3	3	11	11
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	3	3	1	1	4	4
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

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Table GERMANY.2.5.6.7
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Times Admitted to a Hospital for PNH

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	34		14		48	
Mean (SD)	0.1 (0.29)		0.1 (0.36)		0.1 (0.31)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 1		0, 1	
Week 12						
n	34	32	13	13		
Mean (SD)	0.0 (0.00)	-0.1 (0.25)	0.0 (0.00)	-0.2 (0.38)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	-1, 0		
Week 24						
n	31	30	13	13		
Mean (SD)	0.0 (0.00)	-0.1 (0.25)	0.1 (0.28)	-0.1 (0.49)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 1	-1, 1		

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Change from baseline is summarized only for patients who have data at baseline and the specified time point.

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Source: ADaM.ADSL, ADaM.ADQS

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Table GERMANY.2.5.6.7
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Times Admitted to a Hospital for PNH

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	24	24	11	11	35	35
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.2 (0.40)	0.0 (0.00)	-0.1 (0.24)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-1, 0	0, 0	-1, 0
Week 56						
n	19	19	7	7	26	26
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.1 (0.38)	0.0 (0.00)	0.0 (0.20)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-1, 0	0, 0	-1, 0
Week 72						
n	10	10	3	3	13	13
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.6.7
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Background C5 Inhibitor Therapy (Eculizumab or Ravulizumab)
Modified Randomized Set

Times Admitted to a Hospital for PNH

Background C5 Inhibitor Therapy Ravulizumab

Visit/Statistics	D->D (N=36)		P->D (N=14)		Total (N=50)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	8	8	3	3	11	11
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	3	3	1	1	4	4
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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Table GERMANY.2.5.6.8

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Times Gone to an Emergency Room for PNH

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	0.0 (0.00)		0.0 (0.00)		0.0 (0.00)	
Median	0.0		0.0		0.0	
Min, Max	0, 0		0, 0		0, 0	
Week 12						
n	8	8	4	4		
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	0, 0	0, 0	0, 0		
Week 24						
n	8	8	4	4		
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	0, 0	0, 0	0, 0		

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Table GERMANY.2.5.6.8

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Times Gone to an Emergency Room for PNH

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 56						
n	4	4	1	1	5	5
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 72						
n	1	1	0	0	1	1
Mean (SD)	0.0 (NA)	0.0 (NA)			0.0 (NA)	0.0 (NA)
Median	0.0	0.0			0.0	0.0
Min, Max	0, 0	0, 0			0, 0	0, 0

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Table GERMANY.2.5.6.8

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Times Admitted to a Hospital for PNH

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	8		4		12	
Mean (SD)	0.0 (0.00)		0.0 (0.00)		0.0 (0.00)	
Median	0.0		0.0		0.0	
Min, Max	0, 0		0, 0		0, 0	
Week 12						
n	8	8	4	4		
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	0, 0	0, 0	0, 0		
Week 24						
n	8	8	4	4		
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	0, 0	0, 0	0, 0		

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Times Admitted to a Hospital for PNH

Patients Enrolled from Japan Yes

Visit/Statistics	D->D (N=8)		P->D (N=4)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	6	6	3	3	9	9
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 56						
n	4	4	1	1	5	5
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 72						
n	1	1	0	0	1	1
Mean (SD)	0.0 (NA)	0.0 (NA)			0.0 (NA)	0.0 (NA)
Median	0.0	0.0			0.0	0.0
Min, Max	0, 0	0, 0			0, 0	0, 0

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Table GERMANY.2.5.6.8

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No])
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	42		18		60	
Mean (SD)	0.1 (0.30)		0.1 (0.32)		0.1 (0.30)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 1		0, 1	
Week 12						
n	42	38	19	16		
Mean (SD)	0.0 (0.00)	-0.1 (0.31)	0.0 (0.00)	-0.1 (0.34)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	-1, 0		
Week 24						
n	36	33	18	15		
Mean (SD)	0.0 (0.17)	-0.1 (0.29)	0.0 (0.00)	-0.1 (0.35)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 1	-1, 0	0, 0	-1, 0		

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Table GERMANY.2.5.6.8

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Times Gone to an Emergency Room for PNH

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	32	31	18	15	50	46
Mean (SD)	0.0 (0.00)	-0.1 (0.25)	0.0 (0.00)	-0.1 (0.35)	0.0 (0.00)	-0.1 (0.28)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	-1, 0	0, 0	-1, 0
Week 56						
n	24	23	15	14	39	37
Mean (SD)	0.0 (0.20)	0.0 (0.37)	0.0 (0.00)	-0.1 (0.27)	0.0 (0.16)	-0.1 (0.33)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 1	-1, 1	0, 0	-1, 0	0, 1	-1, 1
Week 72						
n	15	14	10	8	25	22
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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Table GERMANY.2.5.6.8

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Times Gone to an Emergency Room for PNH

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	13	12	6	5	19	17
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	4	4	1	1	5	5
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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Table GERMANY.2.5.6.8

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Times Admitted to a Hospital for PNH

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	42		18		60	
Mean (SD)	0.1 (0.42)		0.3 (0.57)		0.2 (0.47)	
Median	0.0		0.0		0.0	
Min, Max	0, 2		0, 2		0, 2	
Week 12						
n	42	38	19	16		
Mean (SD)	0.1 (0.46)	-0.1 (0.66)	0.3 (0.65)	-0.1 (0.85)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 3	-2, 3	0, 2	-2, 2		
Week 24						
n	36	33	18	15		
Mean (SD)	0.0 (0.17)	-0.1 (0.33)	0.1 (0.32)	-0.2 (0.68)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 1	-1, 0	0, 1	-2, 1		

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:49:59

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Table GERMANY.2.5.6.8

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Times Admitted to a Hospital for PNH

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	32	31	18	15	50	46
Mean (SD)	0.1 (0.35)	0.0 (0.55)	0.0 (0.00)	-0.3 (0.62)	0.0 (0.28)	-0.1 (0.58)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 2	-2, 2	0, 0	-2, 0	0, 2	-2, 2
Week 56						
n	24	23	15	14	39	37
Mean (SD)	0.0 (0.00)	0.0 (0.21)	0.2 (0.77)	-0.1 (0.83)	0.1 (0.48)	-0.1 (0.52)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 3	-2, 2	0, 3	-2, 2
Week 72						
n	15	14	10	8	25	22
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.4 (0.74)	0.0 (0.00)	-0.1 (0.47)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-2, 0	0, 0	-2, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.6.8

Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension by the Randomization Stratification Factors of Japanese Patients (Patients Enrolled from Japan [Yes/No]) Modified Randomized Set

Times Admitted to a Hospital for PNH

Patients Enrolled from Japan No

Visit/Statistics	D->D (N=49)		P->D (N=22)		Total (N=71)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	13	12	6	5	19	17
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.4 (0.89)	0.0 (0.00)	-0.1 (0.49)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-2, 0	0, 0	-2, 0
Week 104						
n	4	4	1	1	5	5
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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Table GERMANY.2.5.6.9
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Race
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	0.0 (0.21)		0.0 (0.00)		0.0 (0.18)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 0		0, 1	
Week 12						
n	22	22	10	10		
Mean (SD)	0.0 (0.00)	0.0 (0.21)	0.0 (0.00)	0.0 (0.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	0, 0		
Week 24						
n	21	21	9	9		
Mean (SD)	0.0 (0.00)	0.0 (0.22)	0.0 (0.00)	0.0 (0.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	0, 0		

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This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Race
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	7	7	26	26
Mean (SD)	0.0 (0.00)	-0.1 (0.23)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.20)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	0, 0	0, 0	-1, 0
Week 56						
n	15	15	5	5	20	20
Mean (SD)	0.0 (0.00)	-0.1 (0.26)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	-0.1 (0.22)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	0, 0	0, 0	-1, 0
Week 72						
n	8	8	3	3	11	11
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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Table GERMANY.2.5.6.9
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Race
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	2	2	8	8
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	2	2	0	0	2	2
Mean (SD)	0.0 (0.00)	0.0 (0.00)			0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0			0.0	0.0
Min, Max	0, 0	0, 0			0, 0	0, 0

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Table GERMANY.2.5.6.9
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Race
Modified Randomized Set

Times Admitted to a Hospital for PNH

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		10		32	
Mean (SD)	0.0 (0.00)		0.0 (0.00)		0.0 (0.00)	
Median	0.0		0.0		0.0	
Min, Max	0, 0		0, 0		0, 0	
Week 12						
n	22	22	10	10		
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	0, 0	0, 0	0, 0		
Week 24						
n	21	21	9	9		
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.1 (0.33)	0.1 (0.33)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	0, 0	0, 1	0, 1		

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This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

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Table GERMANY.2.5.6.9
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by Race
Modified Randomized Set

Times Admitted to a Hospital for PNH

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	19	19	7	7	26	26
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 56						
n	15	15	5	5	20	20
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 72						
n	8	8	3	3	11	11
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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Table GERMANY.2.5.6.9
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Race
Modified Randomized Set

Times Admitted to a Hospital for PNH

Asian

Visit/Statistics	D->D (N=22)		P->D (N=10)		Total (N=32)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	6	2	2	8	8
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	2	2	0	0	2	2
Mean (SD)	0.0 (0.00)	0.0 (0.00)			0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0			0.0	0.0
Min, Max	0, 0	0, 0			0, 0	0, 0

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Table GERMANY.2.5.6.9
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Race
Modified Randomized Set

Times Gone to an Emergency Room for PNH

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		7		29	
Mean (SD)	0.1 (0.35)		0.3 (0.49)		0.2 (0.38)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 1		0, 1	
Week 12						
n	21	18	10	7		
Mean (SD)	0.0 (0.00)	-0.2 (0.38)	0.0 (0.00)	-0.3 (0.49)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	-1, 0		
Week 24						
n	19	16	9	6		
Mean (SD)	0.1 (0.23)	-0.1 (0.34)	0.0 (0.00)	-0.3 (0.52)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 1	-1, 0	0, 0	-1, 0		

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

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Table GERMANY.2.5.6.9
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Race
Modified Randomized Set

Times Gone to an Emergency Room for PNH

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	14	13	9	6	23	19
Mean (SD)	0.0 (0.00)	-0.1 (0.28)	0.0 (0.00)	-0.3 (0.52)	0.0 (0.00)	-0.2 (0.37)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	-1, 0	0, 0	-1, 0
Week 56						
n	10	9	6	5	16	14
Mean (SD)	0.0 (0.00)	-0.1 (0.33)	0.0 (0.00)	-0.2 (0.45)	0.0 (0.00)	-0.1 (0.36)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	-1, 0	0, 0	-1, 0
Week 72						
n	7	6	5	3	12	9
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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by Race
Modified Randomized Set

Times Gone to an Emergency Room for PNH

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	5	2	1	8	6
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.0 (NA)	0.0 (NA)			0.0 (NA)	0.0 (NA)
Median	0.0	0.0			0.0	0.0
Min, Max	0, 0	0, 0			0, 0	0, 0

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Table GERMANY.2.5.6.9
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Race
Modified Randomized Set

Times Admitted to a Hospital for PNH

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	22		7		29	
Mean (SD)	0.2 (0.39)		0.3 (0.49)		0.2 (0.41)	
Median	0.0		0.0		0.0	
Min, Max	0, 1		0, 1		0, 1	
Week 12						
n	21	18	10	7		
Mean (SD)	0.1 (0.65)	0.0 (0.84)	0.1 (0.32)	-0.3 (0.49)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 3	-1, 3	0, 1	-1, 0		
Week 24						
n	19	16	9	6		
Mean (SD)	0.0 (0.00)	-0.2 (0.40)	0.0 (0.00)	-0.3 (0.52)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	-1, 0	0, 0	-1, 0		

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Times Admitted to a Hospital for PNH

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	14	13	9	6	23	19
Mean (SD)	0.1 (0.53)	0.1 (0.64)	0.0 (0.00)	-0.3 (0.52)	0.1 (0.42)	-0.1 (0.62)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 2	-1, 2	0, 0	-1, 0	0, 2	-1, 2
Week 56						
n	10	9	6	5	16	14
Mean (SD)	0.0 (0.00)	-0.1 (0.33)	0.0 (0.00)	-0.2 (0.45)	0.0 (0.00)	-0.1 (0.36)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-1, 0	0, 0	-1, 0	0, 0	-1, 0
Week 72						
n	7	6	5	3	12	9
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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by Race
Modified Randomized Set

Times Admitted to a Hospital for PNH

White

Visit/Statistics	D->D (N=28)		P->D (N=11)		Total (N=39)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	6	5	2	1	8	6
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	1	1	0	0	1	1
Mean (SD)	0.0 (NA)	0.0 (NA)			0.0 (NA)	0.0 (NA)
Median	0.0	0.0			0.0	0.0
Min, Max	0, 0	0, 0			0, 0	0, 0

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Table GERMANY.2.5.6.9
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by Race
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	6		5		11	
Mean (SD)	0.0 (0.00)		0.0 (0.00)		0.0 (0.00)	
Median	0.0		0.0		0.0	
Min, Max	0, 0		0, 0		0, 0	
Week 12						
n	7	6	3	3		
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	0, 0	0, 0	0, 0		
Week 24						
n	4	4	4	4		
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 0	0, 0	0, 0	0, 0		

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Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	5	5	5	5	10	10
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 56						
n	3	3	5	5	8	8
Mean (SD)	0.3 (0.58)	0.3 (0.58)	0.0 (0.00)	0.0 (0.00)	0.1 (0.35)	0.1 (0.35)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 1	0, 1	0, 0	0, 0	0, 1	0, 1
Week 72						
n	1	1	2	2	3	3
Mean (SD)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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by Race
Modified Randomized Set

Times Gone to an Emergency Room for PNH

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	1	1	2	2	3	3
Mean (SD)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.0 (NA)	0.0 (NA)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

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Times Admitted to a Hospital for PNH

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Baseline						
n	6		5		11	
Mean (SD)	0.3 (0.82)		0.6 (0.89)		0.5 (0.82)	
Median	0.0		0.0		0.0	
Min, Max	0, 2		0, 2		0, 2	
Week 12						
n	7	6	3	3		
Mean (SD)	0.0 (0.00)	-0.3 (0.82)	1.3 (1.15)	0.3 (2.08)		
Median	0.0	0.0	2.0	1.0		
Min, Max	0, 0	-2, 0	0, 2	-2, 2		
Week 24						
n	4	4	4	4		
Mean (SD)	0.3 (0.50)	-0.3 (0.50)	0.3 (0.50)	-0.5 (1.00)		
Median	0.0	0.0	0.0	0.0		
Min, Max	0, 1	-1, 0	0, 1	-2, 0		

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by Race
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Times Admitted to a Hospital for PNH

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 40						
n	5	5	5	5	10	10
Mean (SD)	0.0 (0.00)	-0.4 (0.89)	0.0 (0.00)	-0.6 (0.89)	0.0 (0.00)	-0.5 (0.85)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	-2, 0	0, 0	-2, 0	0, 0	-2, 0
Week 56						
n	3	3	5	5	8	8
Mean (SD)	0.0 (0.00)	0.0 (0.00)	0.6 (1.34)	0.0 (1.41)	0.4 (1.06)	0.0 (1.07)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 3	-2, 2	0, 3	-2, 2
Week 72						
n	1	1	2	2	3	3
Mean (SD)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	-1.5 (0.71)	0.0 (0.00)	-1.0 (1.00)
Median	0.0	0.0	0.0	-1.5	0.0	-1.0
Min, Max	0, 0	0, 0	0, 0	-2, -1	0, 0	-2, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:49:59

Data cutoff date of 31Mar2023

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/alxn/a2040-pnh-301/a2040-pnh-301-gba/Files/tables/production/programs/t-hru-summary.sas

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Table GERMANY.2.5.6.9
Summary of Actual Values and Change from Baseline in HRU During Treatment Period 1 and Period 2 and Long Term Extension
by Race
Modified Randomized Set

Times Admitted to a Hospital for PNH

Others or Unknown

Visit/Statistics	D->D (N=7)		P->D (N=5)		Total (N=12)	
	Actual Value	Change from Baseline	Actual Value	Change from Baseline	Actual Value	Change from Baseline
Week 88						
n	1	1	2	2	3	3
Mean (SD)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	-1.0 (1.41)	0.0 (0.00)	-0.7 (1.15)
Median	0.0	0.0	0.0	-1.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	-2, 0	0, 0	-2, 0
Week 104						
n	1	1	1	1	2	2
Mean (SD)	0.0 (NA)	0.0 (NA)	0.0 (NA)	0.0 (NA)	0.0 (0.00)	0.0 (0.00)
Median	0.0	0.0	0.0	0.0	0.0	0.0
Min, Max	0, 0	0, 0	0, 0	0, 0	0, 0	0, 0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month. Only How many times gone to an Emergency Room for treatment of PNH and How many times admitted to a Hospital for treatment of PNH were reported.

Patients were randomized to Danicopan or Placebo for 12 weeks as Treatment Period 1. At Week 12, patients randomized to Placebo were switched to Danicopan for an additional 12 weeks as Treatment Period 2 and patients randomized to Danicopan continued on Danicopan during Treatment Period 2. At the end of the Treatment Period 2 (Week 24), patients entered the Long Term Extension (LTE) Period and continue to receive Danicopan.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D: Placebo at Treatment Period 1 and Danicopan at Treatment Period 2.

Change from baseline is summarized only for patients who have data at baseline and the specified time point.

Baseline is defined as the last non-missing value prior to the first dose of study drug.

Actual values for Total group is summarized only for baseline and the time points from Long Term Extension (LTE).

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:49:59

Data cutoff date of 31Mar2023

FINAL

/alxn/a2040-pnh-301/a2040-pnh-301-gba/Files/tables/production/programs/t-hru-summary.sas

Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

3 Sicherheit

3.4 Hospitalisierungen anhand des Healthcare Resource Utilization (HRU)

3.4.3 Anteil hospitalisierter Patienten anhand des HRU innerhalb TP1

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Table GERMANY.2.5.6.11
HRU: Proportion of Patients Gone to an Emergency Room for Treatment of PNH at Week 12
Modified Randomized Set

Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value
--	--	----------------	----------------	----------------	---------

No Records Meet the Criteria for this Table.

HRU is Resource Utilization Patient Questionnaire.
This questionnaire asks about health care services the patient may have received during the past month.
n = number of patients gone to an emergency room for treatment of PNH at week 12, % = n/N*100.
RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.
P-value is from the fisher exact test for treatment comparison.
95% CI for each treatment group is computed using Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.
Source: ADaM.ADSL, ADaM.ADQS
Run Date: 2024-04-26T15:50:01
/alxn/a2040-pnh-301/a2040-pnh-301-gba/Files/tables/production/programs/t-hru.sas

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Table GERMANY.2.5.6.12
HRU: Proportion of Patients Been Admitted to a Hospital for PNH at Week 12
Modified Randomized Set

Danicopan (N=57) n (%) (95% CI)	Placebo (N=26) n (%) (95% CI)	RR (95% CI)	OR (95% CI)	RD (95% CI)	p-value
1 (1.8) (0.0, 9.4)	3 (11.5) (2.4, 30.2)	0.15 (0.01, 1.46)	0.14 (0.00, 1.85)	-0.10 (-0.28, 0.01)	0.0888

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

n = number of patients been admitted to a hospital for PNH at week 12, % = n/N*100.

RR = Relative Risk, OR = Odds Ratio, RD = Risk Difference.

P-value is from the fisher exact test for treatment comparison.

95% CI for each treatment group is computed using Clopper-Pearson method. Exact unconditional approach is used for calculating the 95% CI for RD, RR and OR.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2024-04-26T15:50:01

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/alxn/a2040-pnh-301/a2040-pnh-301-gba/Files/tables/production/programs/t-hru.sas

Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

3 Sicherheit

3.4 Hospitalisierungen anhand des Healthcare Resource Utilization (HRU)

3.4.4 Listing der hospitalisierten Patienten anhand des HRU

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0055-101	Day 1	2021-05-18	1	3	1	1	1	6
	Week 12	2021-08-10	85	2	0	0	0	0
	Early Termination	2021-11-03	170	1	1	0	1	1

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0098-101	Day 1	2021-10-06	1	1	0	0	0	0
	Week 12	2021-12-28	84	3	0	0	0	0
	Week 24	2022-03-23	169	1	0	0	0	0
	Week 40	2022-07-13	281	1	0	0	0	0
	Week 56	2022-11-02	393	1	0	0	0	0
	Week 72	2023-02-22	505	1	0	0	0	1

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0123-101	Day 1	2022-05-05	1	0	0	0	0	0
	Week 12	2022-07-29	86	0	0	0	0	0
	Week 24	2022-10-20	169	0	0	0	1	0
	Week 40	2023-02-09	281	1	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0157-101	Day 1	2021-01-06	1	1	0	0	6	0
	Week 12	2021-03-31	85	0	0	0	0	0
	Week 24	2021-06-23	169	0	0	0	0	0
	Week 40	2021-10-13	281	0	0	0	0	0
	Week 56	2022-02-02	393	0	0	0	0	0
	Week 72	2022-06-01	512	0	0	0	0	0
	Week 88	2022-09-21	624	0	0	0	0	0
	Week 96 - EOT -	2023-01-23	748	0	0	0	0	0
	34.031							

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0157-102	Day 1	2022-03-02	1	0	0	0	4	0
	Week 12	2022-05-25	85	0	0	0	4	0
	Week 24	2022-08-17	169	0	0	0	4	0
	Week 40	2022-12-07	281	1	0	0	3	0
	Week 56	2023-03-31	395	2	1	0	7	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0158-101	Week 12	2022-05-04	86	0	0	0	5	30
	Week 24	2022-07-26	169	2	0	0	3	28
	Week 56	2023-03-10	396	0	0	0	0	30

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0223-102	Day 1	2021-04-16	1	1	0	0	9	1
	Week 12	2021-07-09	85	0	0	0	4	0
	Week 24	2021-10-01	169	0	0	0	0	1
	Week 40	2022-01-21	281	1	0	0	0	3
	Early Termination	2022-02-11	302	0	0	0	0	2

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0263-102	Day 1	2021-02-17	1	0	0	0	0	0
	Week 12	2021-05-12	85	0	0	0	0	0
	Week 40	2021-11-24	281	0	0	0	0	0
	Week 56	2022-03-16	393	0	0	0	0	0
	Week 72	2022-07-08	507	0	0	0	0	0
	Week 88	2022-10-24	615	0	0	0	0	0
	Week 104	2023-02-14	728	0	0	0	0	0
	Week 104 - EOT -	2023-03-28	770	0	0	0	0	0
	35.011							

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0263-104	Day 1	2021-11-22	1	1	0	0	30	5
	Week 12	2022-02-14	85	0	0	0	27	0
	Week 24	2022-05-09	169	0	0	0	30	0
	Week 40	2022-09-06	289	0	0	0	0	0
	Early Termination	2022-12-19	393	0	0	0	5	7

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0292-101	Day 1	2021-12-27	-1	2	0	0	0	0
	Week 12	2022-03-21	84	3	0	0	0	0
	Week 40	2022-10-03	280	1	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

RAQNS data cutoff date of 31Mar2023

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/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0292-102	Day 1	2022-07-01	1	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0313-101	Day 1	2022-08-10	1	0	0	0	0	0
	Week 12	2022-11-01	84	0	0	0	0	0
	Week 24	2023-01-25	169	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0336-101	Day 1	2022-04-07	1	2	0	2	1	
	Week 12	2022-06-29	84	0	0	0	0	0
	Week 24	2022-09-22	169	1	0	1	0	0
	Week 40	2023-01-12	281	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0393-101	Day 1	2022-01-03	1	0	0	0	0	0
	Week 12	2022-03-28	85	0	0	0	0	0
	Week 24	2022-06-20	169	2	0	0	0	0
	Week 40	2022-10-12	283	0	0	0	0	0
	Week 56	2023-01-31	394	0	0	0	0	0
	Week 18 -	2022-05-12	130	0	0	0	0	0
	Unscheduled - 15.01							
	Week 32 -	2022-08-15	225	0	0	0	0	0
	Unscheduled - 21.01							

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

RAQNS data cutoff date of 31Mar2023

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/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0395-101	Day 1	2021-06-03	1	0	0	1	0	0
	Early Termination	2021-07-29	57	0	0	0	1	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0395-102	Day 1	2021-06-17	1	0	0	0	0	0
	Week 12	2021-09-09	85	0	0	0	0	0
	Week 24	2021-12-02	169	0	0	0	0	0
	Week 40	2022-03-24	281	0	0	0	0	0
	Week 56	2022-07-14	393	0	0	0	0	0
	Week 72	2022-11-03	505	0	0	0	0	0
	Week 88	2023-02-22	616	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0422-102	Day 1	2021-04-15	1	2	0	0	0	0
	Week 12	2021-07-08	85	2	0	0	0	0
	Week 24	2021-09-30	169	2	0	0	0	0
	Week 40	2022-01-20	281	2	0	0	0	0
	Week 56	2022-05-11	392	1	0	0	0	0
	Week 72	2022-08-31	504	1	0	0	0	0
	Week 88	2022-12-26	621	0	0	0	0	0
	Week 96 - EOT -	2023-03-16	701	0	0	0	0	0
	34.021							

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0422-103	Day 1	2021-04-29	1	1	0	0	0	0
	Week 12	2021-07-22	85	1	0	0	0	0
	Week 24	2021-10-14	169	1	0	0	0	0
	Week 40	2022-02-03	281	0	0	0	0	0
	Week 56	2022-05-25	392	1	0	0	0	0
	Week 72	2022-09-15	505	1	0	0	0	0
	Week 88	2022-12-28	609	0	0	0	0	0
	Week 96 - EOT -	2023-03-16	687	0	0	0	0	0
	34.021							

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0422-104	Day 1	2021-07-28	1	1	0	0	0	0
	Week 12	2021-10-20	85	1	0	0	0	0
	Week 24	2022-01-12	169	0	0	0	0	0
	Week 40	2022-05-04	281	0	0	0	0	0
	Week 56	2022-08-22	391	0	0	0	0	0
	Week 72	2022-12-12	503	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0422-105	Day 1	2021-09-08	1	0	0	0	0	0
	Week 12	2021-12-01	85	1	0	0	0	0
	Week 24	2022-02-23	169	0	0	0	0	0
	Week 40	2022-06-15	281	0	0	0	0	0
	Week 56	2022-10-05	393	0	0	0	0	0
	Week 72	2023-01-26	506	0	0	0	0	0
	Week 72 - EOT - 31.011	2023-03-22	561	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0422-106	Day 1	2022-01-20	1	0	0	0	0	0
	Week 12	2022-04-14	85	1	0	0	0	0
	Week 24	2022-07-06	168	0	0	0	0	0
	Week 40	2022-10-27	281	0	0	0	0	0
	Week 56	2023-02-16	393	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0429-101	Day 1	2022-01-26	1	0	1	1	0	0
	Week 12	2022-04-20	85	2	0	0	0	0
	Week 24	2022-07-13	169	2	0	0	0	0
	Week 40	2022-11-02	281	0	0	0	0	0
	Week 56	2023-02-24	395	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0432-102	Week 24	2021-10-08	169	0	0	0	0	0
	Week 40	2022-01-28	281	0	0	0	0	0
	Week 88	2022-12-30	617	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0432-103	Week 12	2021-07-22	85	0	0	0	0	0
	Early Termination	2022-08-08	467	1	0	0	4	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0432-106	Day 1	2021-07-06	1	1	0	0	0	0
	Week 12	2021-09-29	86	3	0	3	0	0
	Early Termination	2022-04-12	281	3	0	2	1	

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0432-107	Day 1	2021-10-11	1	0	0	0	0	0
	Week 72	2023-02-23	501	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0432-109	Day 1	2022-08-04	1	4	1	1	0	3
	Week 12	2022-10-27	85	0	0	0	0	30
	Week 24	2023-01-19	169	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0432-110	Day 1	2022-05-12	1	0	0	0	0	0
	Week 24	2022-10-26	168	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0523-101	Day 1	2021-06-01	1	2	0	0	20	0
	Week 12	2021-08-24	85	2	0	0	0	0
	Week 24	2021-11-16	169	2	0	0	0	0
	Week 40	2022-03-09	282	2	0	0	0	0
	Week 56	2022-07-01	396	0	0	0	0	0
	Week 72	2022-10-18	505	0	0	0	0	0
	Week 88	2023-02-07	617	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0555-102	Day 1	2021-07-08	1	0	0	0	0	0
	Week 12	2021-09-30	85	2	0	0	0	0
	Week 24	2021-12-23	169	2	0	0	0	0
	Week 40	2022-04-14	281	1	0	0	0	0
	Week 56	2022-08-04	393	0	0	0	0	0
	Week 72	2022-11-24	505	0	0	0	0	0
	Week 80 - EOT - 32.021	2023-02-16	589	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0555-104	Day 1	2021-12-16	1	1	0	0	0	0
	Week 12	2022-03-10	85	2	0	0	0	0
	Week 24	2022-06-02	169	2	0	0	0	0
	Week 40	2022-09-22	281	0	0	0	2	0
	Week 56	2023-01-12	393	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0605-101	Day 1	2022-05-17	1	0	0	0	0	0
	Week 12	2022-08-09	85	0	0	0	0	0
	Week 24	2022-11-01	169	0	0	0	0	0
	Week 40	2023-02-23	283	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0610-101	Day 1	2021-05-25	1	2	0	0	0	0
	Week 12	2021-08-17	85	2	0	0	0	0
	Week 24	2021-11-09	169	2	0	0	0	0
	Week 40	2022-02-28	280	1	0	0	0	0
	Week 56	2022-06-21	393	1	0	0	0	0
	Week 72	2022-10-11	505	1	0	0	0	0
	Week 88	2023-02-07	624	0	0	0	0	0
	Week 88 - EOT - 33.011	2023-02-28	645	1	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0610-102	Day 1	2021-07-15	1	2	0	0	0	0
	Week 12	2021-10-08	86	2	0	0	0	0
	Week 40	2022-04-21	281	1	0	0	0	0
	Week 56	2022-08-11	393	1	0	0	0	0
	Week 72	2022-12-01	505	1	0	0	0	0
	Week 80 - EOT - 32.011	2023-02-23	589	1	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0629-101	Day 1	2022-01-17	1	0	1	0	0	0
	Week 12	2022-04-11	85	0	0	0	0	0
	Week 24	2022-07-05	170	0	0	0	0	0
	Week 40	2022-10-25	282	0	0	0	0	0
	Week 56	2023-02-13	393	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0629-102	Day 1	2022-04-07	1	0	0	0	0	0
	Week 12	2022-06-29	84	0	0	0	0	0
	Week 24	2022-09-21	168	0	0	0	0	0
	Week 40	2023-01-12	281	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0629-103	Day 1	2022-04-05	1	0	0	0	0	0
	Week 12	2022-06-28	85	0	0	0	0	0
	Week 24	2022-09-20	169	0	0	0	0	0
	Week 40	2023-01-11	282	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0633-101	Day 1	2021-09-29	1	3	0	0	0	30
	Week 12	2021-12-22	85	1	0	0	0	0
	Week 24	2022-03-16	169	2	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0633-102	Day 1	2021-12-16	1	1	0	0	3	14
	Week 12	2022-03-09	84	2	0	0	35	30
	Week 24	2022-06-01	168	2	0	0	35	21
	Week 40	2022-09-20	279	0	0	0	6	22
	Week 56	2023-01-11	392	0	0	0	5	22

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0692-101	Day 1	2021-05-05	1	1	0	0	0	0
	Week 12	2021-07-28	85	3	0	0	0	0
	Week 24	2021-10-20	169	3	0	0	0	0
	Week 40	2022-02-09	281	0	0	0	0	0
	Week 56	2022-06-01	393	0	0	0	0	0
	Week 72	2022-09-23	507	0	0	0	0	0
	Week 88	2023-01-13	619	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0716-102	Day 1	2021-05-25	1	0	0	0	0	0
	Week 12	2021-08-17	85	0	0	0	0	0
	Week 24	2021-11-09	169	2	0	0	0	0
	Early Termination	2021-12-24	214	1	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0768-101	Day 1	2022-08-09	1	2	0	0	0	0
	Week 12	2022-11-01	85	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0784-101	Day 1	2022-05-13	1	0	0	0	0	0
	Week 12	2022-08-05	85	2	0	0	0	0
	Week 24	2022-10-28	169	2	0	0	0	0
	Week 40	2023-02-17	281	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0801-102	Day 1	2021-11-18	1	2	0	0	0	0
	Week 12	2022-02-10	85	0	0	0	0	0
	Week 24	2022-05-06	170	2	0	0	0	0
	Week 40	2022-08-26	282	0	0	0	0	0
	Week 56	2022-12-15	393	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0801-103	Day 1	2021-11-26	1	3	0	0	0	0
	Week 12	2022-02-18	85	2	0	0	0	0
	Week 24	2022-05-13	169	2	0	0	0	8
	Week 40	2022-09-02	281	0	0	0	0	0
	Week 56	2022-12-23	393	2	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
1257-101	Day 1	2022-07-06	1	5	0	0	20	30
	Week 12	2022-09-28	85	5	0	0	0	1
	Week 24	2022-12-21	169	2	0	0	2	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
1465-102	Day 1	2022-01-05	1	2	0	0	0	0
	Week 12	2022-03-30	85	2	0	0	0	0
	Week 24	2022-06-21	168	2	0	0	0	0
	Week 40	2022-10-12	281	1	0	0	0	0
	Week 56	2023-02-01	393	1	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
1683-101	Day 1	2021-06-22	1	0	0	0	0	30
	Week 12	2021-09-14	85	0	0	0	0	30
	Week 24	2021-12-07	169	0	0	0	0	30
	Week 40	2022-03-29	281	0	0	0	0	0
	Week 56	2022-07-19	393	1	0	0	1	0
	Week 72	2022-11-10	507	0	0	0	0	0
	Week 88	2023-03-02	619	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
1711-103	Day 1	2022-03-01	1	0	0	0	0	0
	Week 12	2022-05-24	85	0	0	0	0	0
	Week 24	2022-08-16	169	0	0	0	0	0
	Week 40	2022-12-06	281	0	0	0	0	0
	Week 56	2023-03-28	393	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
1711-104	Day 1	2022-02-17	1	2	0	0	0	0
	Week 12	2022-05-12	85	0	0	0	0	0
	Week 24	2022-08-04	169	0	0	0	0	0
	Week 40	2022-11-24	281	0	0	0	0	0
	Week 56	2023-03-16	393	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
1716-101	Day 1	2021-04-14	1	1	0	0	30	0
	Week 12	2021-07-07	85	0	0	0	2	0
	Week 24	2021-09-29	169	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
1716-102	Day 1	2021-08-27	1	0	0	0	0	0
	Week 12	2021-11-17	83	0	0	0	3	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
1716-103	Week 12	2021-11-17	85	1	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
1963-101	Day 1	2022-08-30	2	2	0	0	30	0
	Week 12	2022-11-24	88	0	0	0	0	0
	Week 24	2023-02-13	169	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Danicopan

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
1989-102	Day 1	2022-06-16	1	2	0	0	1	0
	Week 12	2022-09-09	86	1	0	0	0	0
	Week 24	2022-12-01	169	1	0	0	0	0
	Week 40	2023-03-23	281	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0086-101	Day 1	2022-06-30	1	3	0	0	0	0
	Week 12	2022-09-22	85	3	0	0	0	0
	Early Termination	2022-12-02	156	2	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0123-102	Day 1	2021-10-12	1	2	0	0	0	
	Week 12	2022-01-04	85	0	0	0	1	0
	Week 24	2022-03-29	169	0	0	0	0	0
	Week 40	2022-07-19	281	0	0	0	0	0
	Week 56	2022-11-09	394	2	0	0	0	0
	Week 72	2023-02-28	505	2	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0263-101	Day 1	2021-02-23	1	2	0	0	0	50
	Week 24	2021-08-10	169	0	0	0	0	0
	Week 40	2021-11-30	281	0	0	0	30	0
	Week 56	2022-03-22	393	0	0	0	0	0
	Week 88	2022-11-02	618	0	0	0	0	0
	Week 104	2023-02-21	729	0	0	0	0	0
	Week 104 - EOT - 35.021	2023-03-07	743	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0282-101	Day 1	2021-02-11	1	1	0	0	0	0
	Week 12	2021-05-06	85	0	0	0	0	0
	Week 56	2022-03-09	392	0	0	0	0	0
	Week 72	2022-06-30	505	2	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0368-101	Day 1	2021-07-30	1	2	0	0	20	0
	Week 12	2021-10-20	83	0	0	0	0	0
	Week 24	2022-01-13	168	1	0	1	0	0
	Week 40	2022-05-03	278	0	0	0	7	0
	Week 56	2022-08-25	392	0	0	0	0	0
	Week 72	2022-12-15	504	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0422-101	Day 1	2021-06-30	1	0	0	0	0	0
	Week 12	2021-09-23	86	1	0	0	0	0
	Week 24	2021-12-15	169	0	0	0	0	0
	Week 40	2022-04-07	282	0	0	0	0	0
	Week 56	2022-07-27	393	0	0	0	0	0
	Week 72	2022-11-17	506	0	0	0	0	0
	Week 80 - EOT - 32.021	2023-03-08	617	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0429-102	Day 1	2022-02-04	1	2	1	1	1	7
	Week 12	2022-04-26	82	1	0	0	0	2
	Week 24	2022-07-19	166	2	0	0	0	2
	Week 40	2022-11-08	278	0	0	0	0	0
	Week 56	2023-02-28	390	1	0	0	0	2

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0432-104	Week 12	2021-10-21	85	0	0	1	3	0
	Week 24	2022-01-13	169	2	0	0	3	0
	Week 40	2022-05-05	281	0	0	0	0	0
	Week 72	2022-12-16	506	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0432-105	Week 12	2021-08-12	85	0	0	0	0	0
	Week 24	2021-11-04	169	0	0	0	0	0
	Week 40	2022-02-24	281	0	0	0	0	0
	Week 72	2022-10-06	505	1	0	0	0	0
	Week 88	2023-01-26	617	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0433-101	Day 1	2022-03-30	1	0	1	1	0	0
	Week 12	2022-06-22	85	1	0	0	0	0
	Week 24	2022-09-14	169	0	0	0	0	0
	Week 40	2023-01-11	288	0	0	0	2	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0466-101	Day 1	2021-04-21	1	1	0	2		0
	Week 12	2021-07-13	84	2	0	0	0	0
	Week 24	2021-10-06	169	0	0	0	0	0
	Week 40	2022-01-24	279	0	0	0	0	0
	Week 56	2022-05-18	393	0	0	0	0	0
	Week 72	2022-09-07	505	0	0	0	0	0
	Week 88	2022-12-28	617	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0555-101	Day 1	2021-05-27	1	3	0	0	5	0
	Week 12	2021-08-19	85	0	0	0	0	0
	Week 24	2021-11-11	169	2	0	0	0	0
	Week 40	2022-03-07	285	1	0	0	0	0
	Week 56	2022-06-23	393	1	0	0	0	0
	Week 72	2022-10-13	505	0	0	0	0	0
	Week 88	2023-02-02	617	0	0	0	0	0
	Week 88 - EOT - 33.011	2023-03-27	670	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0555-103	Day 1	2021-09-28	1	1	0	0	0	0
	Early Termination	2021-12-20	84	2	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0630-101	Day 1	2022-02-10	1	2	0	0	3	0
	Week 12	2022-05-05	85	0	0	0	0	0
	Week 24	2022-07-28	169	2	0	0	0	0
	Week 40	2022-11-17	281	2	0	0	0	0
	Week 56	2023-03-09	393	2	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0692-102	Day 1	2021-05-19	1	4	0	0	0	0
	Week 12	2021-08-10	84	3	0	0	0	0
	Week 24	2021-11-03	169	3	0	0	1	0
	Week 40	2022-02-23	281	0	0	0	0	0
	Week 56	2022-06-15	393	0	0	0	0	0
	Week 72	2022-10-05	505	0	0	0	0	0
	Week 88	2023-01-25	617	1	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0712-102	Day 1	2021-12-23	1	1	0	0	0	0
	Week 12	2022-03-17	85	0	0	0	0	0
	Week 24	2022-06-09	169	1	0	0	0	0
	Early Termination	2022-06-23	183	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
0719-101	Day 1	2021-11-24	1	0	0	0	0	0
	Week 12	2022-02-17	86	0	0	0	0	0
	Week 24	2022-05-12	170	0	0	0	0	0
	Week 40	2022-08-30	280	0	0	0	0	0
	Week 56	2022-12-20	392	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
1360-101	Day 1	2022-01-03	1	2	0	0	1	0
	Week 24	2022-06-21	170	3	0	0	0	0
	Week 40	2022-10-18	289	1	0	0	0	0
	Week 56	2023-02-07	401	1	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
1465-101	Day 1	2021-12-22	1	4	0	0	6	0
	Week 12	2022-03-16	85	2	0	0	1	0
	Week 24	2022-06-08	169	2	0	0	0	0
	Week 40	2022-09-28	281	1	0	0	0	0
	Week 56	2023-01-18	393	1	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
1713-102	Week 12	2021-10-13	87	0	0	0	0	0
	Week 24	2022-01-04	170	0	0	0	0	0
	Week 40	2022-04-26	282	0	0	0	0	0
	Week 56	2022-08-16	394	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
1817-101	Day 1	2022-05-05	1	0	0	0	0	0
	Week 12	2022-07-28	85	3	0	0	0	3
	Week 24	2022-10-20	169	2	0	0	2	1
	Week 40	2023-02-10	282	0	0	0	1	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
1836-101	Day 1	2021-06-16	1	2	0	1	0	18
	Week 12	2021-09-08	85	2	0	2	0	18
	Week 24	2021-12-01	169	0	0	1	0	16
	Week 40	2022-03-22	280	0	0	0	0	21
	Week 56	2022-07-19	399	2	0	3	0	22
	Week 72	2022-10-31	503	0	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
1836-102	Day 1	2022-02-22	1	0	0	0	0	21
	Week 12	2022-05-17	85	0	0	2	0	20
	Week 40	2022-11-30	282	0	0	0	0	25
	Week 56	2023-03-22	394	0	0	0	0	

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
1947-101	Day 1	2022-06-24	1	1	0	0	0	0
	Week 12	2022-09-16	85	2	0	0	0	0
	Week 24	2022-12-09	169	3	0	0	0	0
	Week 40	2023-03-31	281	3	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

/alxn/a2040-pnh-301/a2040-pnh-301-raqns/Files/listings/production/programs/l-qshru.sas

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Listing 16.2.6.4
HRU
Modified Randomized Set

Treatment Group: Placebo

Subject ID	Visit	Date	Day	How many times visited the health care provider for treatment of PNH?	How many times gone to an Emergency Room for treatment of PNH?	How many times been admitted to a Hospital for treatment of PNH?	How many times had darkened urine?	How many times miss work as a result of symptoms of PNH?
1989-101	Day 1	2022-04-28	1	1	0	0	0	0
	Week 12	2022-07-21	85	1	0	0	0	0
	Week 24	2022-10-13	169	1	0	0	0	0
	Week 40	2023-02-02	281	1	0	0	0	0

HRU is Resource Utilization Patient Questionnaire.

This questionnaire asks about health care services the patient may have received during the past month.

Source: ADaM.ADSL, ADaM.ADQS

Run Date: 2023-08-18T15:51:19

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

4 Weitere Analysen

4.1 Demographische Charakteristika und Krankheitscharakteristika

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Table 14.1.2.1.1
Demographics and Baseline Characteristics
Safety Set

	Danicopan (N = 57)	Placebo (N = 29)	Total (N = 86)
Sex			
n	57	29	86
Male	23 (40.4)	9 (31.0)	32 (37.2)
Female	34 (59.6)	20 (69.0)	54 (62.8)
Undifferentiated	0	0	0
Unknown	0	0	0
Race			
n	57	29	86
American Indian or Alaska Native	1 (1.8)	0	1 (1.2)
Asian	22 (38.6)	10 (34.5)	32 (37.2)
Black or African American	2 (3.5)	0	2 (2.3)
Native Hawaiian or Other Pacific Islander	0	0	0
White	28 (49.1)	14 (48.3)	42 (48.8)
Other	1 (1.8)	0	1 (1.2)
Not Reported	3 (5.3)	4 (13.8)	7 (8.1)
Unknown	0	1 (3.4)	1 (1.2)
Ethnicity			
n	57	29	86
Hispanic or Latino	6 (10.5)	1 (3.4)	7 (8.1)
Not Hispanic or Latino	46 (80.7)	24 (82.8)	70 (81.4)
Not Reported	5 (8.8)	4 (13.8)	9 (10.5)
Unknown	0	0	0

Percentages are based on number of patients with non-missing values in each treatment group.

Source: ADaM.ADSL

Run Date: 2023-08-18T15:53:21

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Table 14.1.2.1.1
Demographics and Baseline Characteristics
Safety Set

	Danicopan (N = 57)	Placebo (N = 29)	Total (N = 86)
Age (years) at Informed Consent			
n	57	29	86
Mean (SD)	52.8 (17.00)	52.9 (14.34)	52.8 (16.07)
Median	56.0	53.0	54.0
Min, Max	20, 82	29, 77	20, 82
Age Group 1 (years) at Informed Consent			
<65	41 (71.9)	23 (79.3)	64 (74.4)
>=65	16 (28.1)	6 (20.7)	22 (25.6)
Age Group 2 (years) at Informed Consent			
<65	41 (71.9)	23 (79.3)	64 (74.4)
65-74	9 (15.8)	4 (13.8)	13 (15.1)
75-84	7 (12.3)	2 (6.9)	9 (10.5)
>=85	0	0	0
Height (cm)			
n	57	29	86
Mean (SD)	166.39 (10.836)	164.36 (8.971)	165.70 (10.237)
Median	166.00	164.00	164.75
Min, Max	140.0, 207.0	143.0, 184.0	140.0, 207.0
Weight (kg)			
n	57	29	86
Mean (SD)	72.34 (18.055)	66.57 (13.262)	70.39 (16.740)
Median	71.00	63.00	67.00
Min, Max	45.5, 143.2	44.6, 101.0	44.6, 143.2

Percentages are based on number of patients with non-missing values in each treatment group.

Source: ADaM.ADSL

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Table 14.1.2.1.1
Demographics and Baseline Characteristics
Safety Set

	Danicopan (N = 57)	Placebo (N = 29)	Total (N = 86)
BMI (kg/m ²)			
n	57	29	86
Mean (SD)	25.984 (5.3183)	24.592 (4.3398)	25.515 (5.0276)
Median	24.505	24.484	24.494
Min, Max	18.56, 49.26	18.37, 37.10	18.37, 49.26
Transfusion History			
n	57	29	86
<=2 transfusions within 6 months of screening	33 (57.9)	17 (58.6)	50 (58.1)
>2 transfusions within 6 months of screening	24 (42.1)	12 (41.4)	36 (41.9)
Screening Hgb level			
n	57	29	86
<8.5 g/dL	35 (61.4)	17 (58.6)	52 (60.5)
>=8.5 g/dL	22 (38.6)	12 (41.4)	34 (39.5)
Japanese patients			
n	57	29	86
No	49 (86.0)	25 (86.2)	74 (86.0)
Yes	8 (14.0)	4 (13.8)	12 (14.0)

Percentages are based on number of patients with non-missing values in each treatment group.

Source: ADaM.ADSL

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Table 14.1.3.1.1
Disease Characteristics
Safety Set

	Danicopan (N = 57)	Placebo (N = 29)	Total (N = 86)
Age (years) at PNH Diagnosis			
n	57	29	86
Mean (SD)	43.34 (17.296)	42.36 (16.267)	43.00 (16.867)
Median	40.60	43.40	41.95
Min, Max	11.6, 76.4	18.0, 72.7	11.6, 76.4
Method of Initial PNH Diagnosis, n (%)			
Flow Cytometry	41 (71.9)	25 (86.2)	66 (76.7)
Ham's Test	3 (5.3)	1 (3.4)	4 (4.7)
Sucrose Hemolysis Test	2 (3.5)	0	2 (2.3)
Other	11 (19.3)	3 (10.3)	14 (16.3)
Years from Diagnosis to Informed Consent			
n	57	29	86
Mean (SD)	9.95 (9.660)	10.97 (9.479)	10.29 (9.556)
Median	6.80	9.30	7.25
Min, Max	0.9, 49.6	1.2, 39.6	0.9, 49.6
Age (years) at first C5 inhibitor infusion			
n	57	29	86
Mean (SD)	48.26 (16.621)	47.36 (14.813)	47.96 (15.952)
Median	49.90	46.30	47.70
Min, Max	19.5, 76.9	20.5, 74.1	19.5, 76.9

Percentages are based on number of patients with non-missing values in each group.

Baseline is defined as the average of all available assessments prior to the first dose of study drug for Lactate Dehydrogenase (LDH), as the lowest value observed between and including Screening and Day 1 for Hgb and as the last non-missing value prior to the first dose of study drug for other parameters.

Source: ADaM.ADSL, ADaM.ADLB, ADaM.ADQS, ADaM.ADCM

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Table 14.1.3.1.1
Disease Characteristics
Safety Set

	Danicopan (N = 57)	Placebo (N = 29)	Total (N = 86)
Duration (years) from initial C5 inhibitor to first dose of study drug			
n	57	29	86
Mean (SD)	5.13 (3.599)	6.11 (4.219)	5.46 (3.823)
Median	4.26	4.65	4.35
Min, Max	0.6, 15.8	0.7, 16.8	0.6, 16.8
Duration (years) from start of current C5 inhibitor to first dose of study drug			
n	57	29	86
Mean (SD)	3.69 (3.081)	4.32 (3.624)	3.90 (3.266)
Median	3.32	3.66	3.49
Min, Max	0.5, 14.2	0.7, 16.8	0.5, 16.8
Current C5 inhibitor, n (%)			
RAVULIZUMAB	36 (63.2)	15 (51.7)	51 (59.3)
ECULIZUMAB	21 (36.8)	14 (48.3)	35 (40.7)
PNH Clone Size at Baseline			
PNH RBC Type II Clone Size (%)			
n	26	15	41
Mean (SD)	6.485 (9.9375)	4.807 (4.8461)	5.871 (8.4030)
Median	1.300	2.400	1.700
Min, Max	0.10, 36.80	0.10, 14.50	0.10, 36.80

Percentages are based on number of patients with non-missing values in each group.

Baseline is defined as the average of all available assessments prior to the first dose of study drug for Lactate Dehydrogenase (LDH), as the lowest value observed between and including Screening and Day 1 for Hgb and as the last non-missing value prior to the first dose of study drug for other parameters.

Source: ADaM.ADSL, ADaM.ADLB, ADaM.ADQS, ADaM.ADCM

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Table 14.1.3.1.1
Disease Characteristics
Safety Set

	Danicopan (N = 57)	Placebo (N = 29)	Total (N = 86)
PNH RBC Type III Clone Size (%)			
n	39	17	56
Mean (SD)	48.938 (25.1011)	46.224 (28.2960)	48.114 (25.8810)
Median	45.400	46.600	46.000
Min, Max	11.00, 97.30	6.70, 99.90	6.70, 99.90
PNH Granulocyte Clone Size (%)			
n	40	18	58
Mean (SD)	95.353 (8.3844)	93.867 (8.5657)	94.891 (8.3942)
Median	98.600	96.500	98.200
Min, Max	63.50, 100.00	68.40, 100.00	63.50, 100.00
Total PNH RBC Clone Size (Type II + Type III) (%)			
n	26	17	43
Mean (SD)	56.804 (27.7079)	52.512 (30.9830)	55.107 (28.7608)
Median	62.150	52.900	55.700
Min, Max	11.10, 99.10	6.80, 100.00	6.80, 100.00
C3d PNH Type 3 Cells (%)			
n	39	18	57
Mean (SD)	32.128 (18.8408)	29.589 (17.1069)	31.326 (18.1971)
Median	28.000	25.500	28.000
Min, Max	2.20, 77.60	1.60, 68.80	1.60, 77.60

Percentages are based on number of patients with non-missing values in each group.

Baseline is defined as the average of all available assessments prior to the first dose of study drug for Lactate Dehydrogenase (LDH), as the lowest value observed between and including Screening and Day 1 for Hgb and as the last non-missing value prior to the first dose of study drug for other parameters.

Source: ADaM.ADSL, ADaM.ADLB, ADaM.ADQS, ADaM.ADCM

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Table 14.1.3.1.1
Disease Characteristics
Safety Set

	Danicopan (N = 57)	Placebo (N = 29)	Total (N = 86)
MdFI C3d PNH Type 3 Cells			
n	39	18	57
Mean (SD)	20.692 (10.6947)	19.111 (9.4985)	20.193 (10.2738)
Median	18.000	16.000	18.000
Min, Max	9.00, 52.00	3.00, 49.00	3.00, 52.00
Hgb at baseline (g/L)			
n	57	29	86
Mean (SD)	76.7 (9.47)	78.9 (10.11)	77.5 (9.69)
Median	78.0	80.0	78.0
Min, Max	55, 94	54, 93	54, 94
Absolute reticulocyte count at baseline (10 ¹² /L)			
n	57	28	85
Mean (SD)	0.24762 (0.097187)	0.22268 (0.115356)	0.23940 (0.103504)
Median	0.22100	0.19180	0.21130
Min, Max	0.1094, 0.5295	0.0394, 0.5419	0.0394, 0.5419
Lactate Dehydrogenase (LDH) at baseline (U/L)			
n	56	28	84
Mean (SD)	304.00 (123.600)	286.40 (93.138)	298.13 (114.082)
Median	261.00	263.00	261.00
Min, Max	140.0, 809.0	139.0, 522.7	139.0, 809.0

Percentages are based on number of patients with non-missing values in each group.

Baseline is defined as the average of all available assessments prior to the first dose of study drug for Lactate Dehydrogenase (LDH), as the lowest value observed between and including Screening and Day 1 for Hgb and as the last non-missing value prior to the first dose of study drug for other parameters.

Source: ADaM.ADSL, ADaM.ADLB, ADaM.ADQS, ADaM.ADCM

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Table 14.1.3.1.1
Disease Characteristics
Safety Set

	Danicopan (N = 57)	Placebo (N = 29)	Total (N = 86)
FACIT-fatigue scores at baseline			
n	56	28	84
Mean (SD)	34.02 (11.265)	31.68 (10.995)	33.24 (11.165)
Median	36.00	32.00	35.00
Min, Max	6.0, 52.0	12.0, 52.0	6.0, 52.0

Percentages are based on number of patients with non-missing values in each group.

Baseline is defined as the average of all available assessments prior to the first dose of study drug for Lactate Dehydrogenase (LDH), as the lowest value observed between and including Screening and Day 1 for Hgb and as the last non-missing value prior to the first dose of study drug for other parameters.

Source: ADaM.ADSL, ADaM.ADLB, ADaM.ADQS, ADaM.ADCM

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

4 Weitere Analysen

4.2 Patienten Disposition

Post-hoc Analysen Studie ALPHA

Alexion Pharmaceuticals, Inc.
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Table 14.1.1.4.3
Study Disposition
All Randomized Patients

Category	Danicopan (N = 57) n (%)	Placebo (N = 29) n (%)	Total (N = 86) n (%)
Randomized	57 (100)	29 (100)	86 (100)
Treated	57 (100)	29 (100)	86 (100)
Treatment Period 1			
Completed	55 (96.5)	27 (93.1)	82 (95.3)
Discontinued	2 (3.5)	2 (6.9)	4 (4.7)
Ongoing	0	0	0
Discontinued Treatment During Treatment Period 1	2	2	4
Adverse Event	2 (3.5)	1 (3.4)	3 (3.5)
Death	0	0	0
Lost To Follow-up	0	0	0
Non-Compliance With Study Drug	0	0	0
Physician Decision	0	0	0
Pregnancy	0	0	0
Protocol Violation	0	0	0
Study Terminated By Sponsor	0	0	0
Withdrawal By Subject	0	1 (3.4)	1 (1.2)
Lack Of Efficacy	0	0	0
Other	0	0	0

% = n/N*100; Percentages are calculated based on the total number of patients in each Treatment group. <A patient may contribute to more than one disposition category.>

Source: ADaM.ADSL

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Table 14.1.1.4.3
Study Disposition
All Randomized Patients

Category	Danicopan (N = 57) n (%)	Placebo (N = 29) n (%)	Total (N = 86) n (%)
Treatment Period 2			
Entered	55 (96.5)	27 (93.1)	82 (95.3)
Completed	54 (94.7)	26 (89.7)	80 (93.0)
Discontinued	1 (1.8)	1 (3.4)	2 (2.3)
Ongoing	0	0	0
Discontinued Treatment During Treatment Period 2	1	1	2
Adverse Event	1 (1.8)	1 (3.4)	2 (2.3)
Death	0	0	0
Lost To Follow-up	0	0	0
Non-Compliance With Study Drug	0	0	0
Physician Decision	0	0	0
Pregnancy	0	0	0
Protocol Violation	0	0	0
Study Terminated By Sponsor	0	0	0
Withdrawal By Subject	0	0	0
Lack Of Efficacy	0	0	0
Other	0	0	0

% = n/N*100; Percentages are calculated based on the total number of patients in each Treatment group. <A patient may contribute to more than one disposition category.>

Source: ADaM.ADSL

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Table 14.1.1.4.3
Study Disposition
All Randomized Patients

Category	Danicopan (N = 57) n (%)	Placebo (N = 29) n (%)	Total (N = 86) n (%)
Long Term Extension			
Entered	54 (94.7)	26 (89.7)	80 (93.0)
Completed	11 (19.3)	4 (13.8)	15 (17.4)
Discontinued	6 (10.5)	1 (3.4)	7 (8.1)
Ongoing	37 (64.9)	21 (72.4)	58 (67.4)
Completed Long Term Extension Year 1	20 (35.1)	12 (41.4)	32 (37.2)
Discontinued Treatment During Long Term Extension	6	1	7
Adverse Event	0	1 (3.4)	1 (1.2)
Death	0	0	0
Lost To Follow-up	0	0	0
Non-Compliance With Study Drug	1 (1.8)	0	1 (1.2)
Physician Decision	2 (3.5)	0	2 (2.3)
Pregnancy	0	0	0
Protocol Violation	0	0	0
Study Terminated By Sponsor	0	0	0
Withdrawal By Subject	3 (5.3)	0	3 (3.5)
Lack Of Efficacy	0	0	0
Other	0	0	0

% = n/N*100; Percentages are calculated based on the total number of patients in each Treatment group. <A patient may contribute to more than one disposition category.>

Source: ADaM.ADSL

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Table 14.1.1.4.3
Study Disposition
All Randomized Patients

Category	Danicopan (N = 57) n (%)	Placebo (N = 29) n (%)	Total (N = 86) n (%)
Entire Study			
Completed	11 (19.3)	4 (13.8)	15 (17.4)
Discontinued	8 (14.0)	4 (13.8)	12 (14.0)
Ongoing	38 (66.7)	21 (72.4)	59 (68.6)
Discontinued Study at any Point During the Study	8	4	12
Adverse Event	2 (3.5)	2 (6.9)	4 (4.7)
Death	0	0	0
Lost To Follow-up	0	0	0
Non-Compliance With Study Drug	1 (1.8)	0	1 (1.2)
Physician Decision	1 (1.8)	0	1 (1.2)
Pregnancy	0	0	0
Protocol Violation	0	0	0
Study Terminated By Sponsor	0	0	0
Withdrawal By Subject	4 (7.0)	2 (6.9)	6 (7.0)
Lack Of Efficacy	0	0	0
Other	0	0	0

% = n/N*100; Percentages are calculated based on the total number of patients in each Treatment group. <A patient may contribute to more than one disposition category.>

Source: ADaM.ADSL

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Anhang 4-G – Post-hoc Analysen Studie ALPHA; Dritter Datenschnitt vom 31.03.2023

4 Weitere Analysen

4.3 Behandlungsdauer

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Table 14.3.1.1.1
Study Drug Exposure and Treatment Compliance for Treatment Period 1
Safety Set

	Danicopan (N=57)	Placebo (N=29)
Treatment duration (days)		
n	57	29
Mean (SD)	82.8 (7.15)	77.3 (15.71)
Median	84.0	84.0
Min, Max	44.0, 88.0	35.0, 86.0
Treatment duration (days) by dose level		
100 mg		
n	3	
Mean (SD)	56.0 (37.04)	
Median	70.0	
Min, Max	14.0, 84.0	
150 mg		
n	56	
Mean (SD)	71.6 (19.79)	
Median	84.0	
Min, Max	14.0, 87.0	
200 mg		
n	14	
Mean (SD)	39.3 (10.17)	
Median	42.0	
Min, Max	7.0, 48.0	

% = n/N*100. Treatment duration is derived as date of last dose - date of first dose + 1.

Treatment compliance based on actual danicopan dosage is defined as the total danicopan dose amount of received divided by the total danicopan dose amount of expected to be received by patient during the period. Treatment compliance based on tablet counts is defined as the total number of tablets received divided by the total number of tablets expected to be received by patient during the period. Compliance was calculated using available study drug accountability data.

Source: ADaM.ADSL, ADaM.ADEXSUM

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Table 14.3.1.1.1
Study Drug Exposure and Treatment Compliance for Treatment Period 1
Safety Set

	Danicopan (N=57)	Placebo (N=29)
Treatment compliance based on danicopan dosage		
> 100%	16 (28.1)	
≥ 90% to < 100%	40 (70.2)	
≥ 80% to < 90%	1 (1.8)	
≥ 70% to < 80%	0	
≥ 60% to < 70%	0	
≥ 50% to < 60%	0	
≥ 40% to < 50%	0	
≥ 30% to < 40%	0	
≥ 20% to < 30%	0	
≥ 10% to < 20%	0	
< 10%	0	
n	57	
Mean (SD)	98.8 (3.86)	
Median	99.1	
Min, Max	83.9, 109.5	

% = n/N*100. Treatment duration is derived as date of last dose - date of first dose + 1.

Treatment compliance based on actual danicopan dosage is defined as the total danicopan dose amount of received divided by the total danicopan dose amount of expected to be received by patient during the period. Treatment compliance based on tablet counts is defined as the total number of tablets received divided by the total number of tablets expected to be received by patient during the period. Compliance was calculated using available study drug accountability data.

Source: ADaM.ADSL, ADaM.ADEXSUM

RAQNS data cutoff date of 31Mar2023

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Table 14.3.1.1.1
Study Drug Exposure and Treatment Compliance for Treatment Period 1
Safety Set

	Danicopan (N=57)	Placebo (N=29)
Treatment compliance based on tablets counts		
> 100%	12 (21.1)	7 (24.1)
≥ 90% to ≤ 100%	42 (73.7)	21 (72.4)
≥ 80% to < 90%	2 (3.5)	0
≥ 70% to < 80%	1 (1.8)	0
≥ 60% to < 70%	0	0
≥ 50% to < 60%	0	0
≥ 40% to < 50%	0	1 (3.4)
≥ 30% to < 40%	0	0
≥ 20% to < 30%	0	0
≥ 10% to < 20%	0	0
< 10%	0	0
n	57	29
Mean (SD)	98.0 (4.44)	96.6 (9.93)
Median	98.8	99.6
Min, Max	77.3, 108.7	46.5, 100.8

% = n/N*100. Treatment duration is derived as date of last dose - date of first dose + 1.

Treatment compliance based on actual danicopan dosage is defined as the total danicopan dose amount of received divided by the total danicopan dose amount of expected to be received by patient during the period. Treatment compliance based on tablet counts is defined as the total number of tablets received divided by the total number of tablets expected to be received by patient during the period. Compliance was calculated using available study drug accountability data.

Source: ADaM.ADSL, ADaM.ADEXSUM

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Table 14.3.1.1.2
Study Drug Exposure and Treatment Compliance for Treatment Period 2
Safety Set

	D->D (N=55)	P->D (N=27)	Total (N=82)
Treatment duration (days)			
n	55	27	82
Mean (SD)	82.8 (9.44)	83.5 (2.55)	83.0 (7.85)
Median	84.0	84.0	84.0
Min, Max	15.0, 94.0	71.0, 85.0	15.0, 94.0
Treatment duration (days) by dose level			
100 mg			
n	1	0	1
Mean (SD)	28.0 (NA)		28.0 (NA)
Median	28.0		28.0
Min, Max	28.0, 28.0		28.0, 28.0
150 mg			
n	24	27	51
Mean (SD)	68.9 (21.81)	60.6 (21.27)	64.5 (21.72)
Median	84.0	43.0	83.0
Min, Max	15.0, 94.0	41.0, 85.0	15.0, 94.0

% = n/N*100. Treatment duration is derived as date of last dose - date of first dose + 1.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D : Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Treatment compliance based on actual danicopan dosage is defined as the total danicopan dose amount of received divided by the total danicopan dose amount of expected to be received by patient during the period. Treatment compliance based on tablet counts is defined as the total number of tablets received divided by the total number of tablets expected to be received by patient during the period. Compliance was calculated using available study drug accountability data.

Source: ADaM.ADSL, ADaM.ADEXSUM

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Table 14.3.1.1.2
Study Drug Exposure and Treatment Compliance for Treatment Period 2
Safety Set

	D->D (N=55)	P->D (N=27)	Total (N=82)
200 mg			
n	38	15	53
Mean (SD)	75.3 (17.89)	41.3 (3.41)	65.7 (21.70)
Median	84.0	42.0	83.0
Min, Max	27.0, 85.0	29.0, 43.0	27.0, 85.0

% = $n/N \times 100$. Treatment duration is derived as date of last dose - date of first dose + 1.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D : Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Treatment compliance based on actual danicopan dosage is defined as the total danicopan dose amount of received divided by the total danicopan dose amount of expected to be received by patient during the period. Treatment compliance based on tablet counts is defined as the total number of tablets received divided by the total number of tablets expected to be received by patient during the period. Compliance was calculated using available study drug accountability data.

Source: ADaM.ADSL, ADaM.ADEXSUM

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Table 14.3.1.1.2
Study Drug Exposure and Treatment Compliance for Treatment Period 2
Safety Set

	D->D (N=55)	P->D (N=27)	Total (N=82)
Treatment compliance based on danicopan dosage			
> 100%	13 (23.6)	5 (18.5)	18 (22.0)
≥ 90% to ≤ 100%	38 (69.1)	21 (77.8)	59 (72.0)
≥ 80% to < 90%	1 (1.8)	1 (3.7)	2 (2.4)
≥ 70% to < 80%	2 (3.6)	0	2 (2.4)
≥ 60% to < 70%	0	0	0
≥ 50% to < 60%	1 (1.8)	0	1 (1.2)
≥ 40% to < 50%	0	0	0
≥ 30% to < 40%	0	0	0
≥ 20% to < 30%	0	0	0
≥ 10% to < 20%	0	0	0
< 10%	0	0	0
n	55	27	82
Mean (SD)	99.0 (9.88)	98.9 (3.60)	99.0 (8.32)
Median	99.7	99.4	99.6
Min, Max	56.5, 131.7	84.4, 106.6	56.5, 131.7

% = n/N*100. Treatment duration is derived as date of last dose - date of first dose + 1.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D : Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Treatment compliance based on actual danicopan dosage is defined as the total danicopan dose amount of received divided by the total danicopan dose amount of expected to be received by patient during the period. Treatment compliance based on tablet counts is defined as the total number of tablets received divided by the total number of tablets expected to be received by patient during the period. Compliance was calculated using available study drug accountability data.

Source: ADaM.ADSL, ADaM.ADEXSUM

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Table 14.3.1.1.2
Study Drug Exposure and Treatment Compliance for Treatment Period 2
Safety Set

	D->D (N=55)	P->D (N=27)	Total (N=82)
Treatment compliance based on tablets counts			
> 100%	16 (29.1)	5 (18.5)	21 (25.6)
≥ 90% to ≤ 100%	35 (63.6)	21 (77.8)	56 (68.3)
≥ 80% to < 90%	1 (1.8)	1 (3.7)	2 (2.4)
≥ 70% to < 80%	2 (3.6)	0	2 (2.4)
≥ 60% to < 70%	0	0	0
≥ 50% to < 60%	1 (1.8)	0	1 (1.2)
≥ 40% to < 50%	0	0	0
≥ 30% to < 40%	0	0	0
≥ 20% to < 30%	0	0	0
≥ 10% to < 20%	0	0	0
< 10%	0	0	0
n	55	27	82
Mean (SD)	98.8 (9.36)	98.7 (3.46)	98.8 (7.89)
Median	99.7	99.2	99.6
Min, Max	56.4, 126.7	84.4, 104.2	56.4, 126.7

% = n/N*100. Treatment duration is derived as date of last dose - date of first dose + 1.

D->D: Danicopan at Treatment Period 1 and Danicopan at Treatment Period 2; P->D : Placebo at Treatment Period 1 and Danicopan at Treatment Period 2. Treatment compliance based on actual danicopan dosage is defined as the total danicopan dose amount of received divided by the total danicopan dose amount of expected to be received by patient during the period. Treatment compliance based on tablet counts is defined as the total number of tablets received divided by the total number of tablets expected to be received by patient during the period. Compliance was calculated using available study drug accountability data.

Source: ADaM.ADSL, ADaM.ADEXSUM

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Table 14.3.1.1.3
Study Drug Exposure and Treatment Compliance for Long Term Extension
Safety Set

	D->D (N=54)	P->D (N=26)	Total (N=80)
Treatment duration (days)			
n	54	26	80
Mean (SD)	293.9 (156.26)	321.4 (171.00)	302.9 (160.63)
Median	284.5	310.5	288.5
Min, Max	46.0, 601.0	15.0, 611.0	15.0, 611.0
Treatment duration (days) by dose level			
100 mg			
n	0	0	0
Mean (SD)			
Median			
Min, Max			
150 mg			
n	16	13	29
Mean (SD)	206.9 (141.30)	243.8 (183.92)	223.4 (159.82)
Median	194.0	235.0	225.0
Min, Max	29.0, 501.0	4.0, 574.0	4.0, 574.0
200 mg			
n	41	15	56
Mean (SD)	306.4 (156.11)	345.8 (172.21)	317.0 (159.95)
Median	300.0	368.0	313.5
Min, Max	46.0, 601.0	11.0, 611.0	11.0, 611.0

% = n/N*100. Treatment duration is derived as date of last dose - date of first dose + 1.

Treatment compliance based on actual danicopan dosage is defined as the total danicopan dose amount of received divided by the total danicopan dose amount of expected to be received by patient during the period. Treatment compliance based on tablet counts is defined as the total number of tablets received divided by the total number of tablets expected to be received by patient during the period. Compliance was calculated using available study drug accountability data.

Source: ADaM.ADSL, ADaM.ADEXSUM

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Table 14.3.1.1.3
Study Drug Exposure and Treatment Compliance for Long Term Extension
Safety Set

	D->D (N=54)	P->D (N=26)	Total (N=80)
Treatment compliance based on danicopan dosage			
> 100%	12 (22.2)	4 (15.4)	16 (20.0)
≥ 90% to ≤ 100%	35 (64.8)	20 (76.9)	55 (68.8)
≥ 80% to < 90%	4 (7.4)	2 (7.7)	6 (7.5)
≥ 70% to < 80%	0	0	0
≥ 60% to < 70%	0	0	0
≥ 50% to < 60%	0	0	0
≥ 40% to < 50%	0	0	0
≥ 30% to < 40%	0	0	0
≥ 20% to < 30%	1 (1.9)	0	1 (1.3)
≥ 10% to < 20%	0	0	0
< 10%	0	0	0
n	52	26	78
Mean (SD)	97.4 (11.53)	98.2 (5.98)	97.7 (9.99)
Median	99.0	98.8	98.9
Min, Max	27.4, 121.5	86.3, 114.5	27.4, 121.5

% = $n/N \times 100$. Treatment duration is derived as date of last dose - date of first dose + 1.

Treatment compliance based on actual danicopan dosage is defined as the total danicopan dose amount of received divided by the total danicopan dose amount of expected to be received by patient during the period. Treatment compliance based on tablet counts is defined as the total number of tablets received divided by the total number of tablets expected to be received by patient during the period. Compliance was calculated using available study drug accountability data.

Source: ADaM.ADSL, ADaM.ADEXSUM

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Table 14.3.1.1.3
Study Drug Exposure and Treatment Compliance for Long Term Extension
Safety Set

	D->D (N=54)	P->D (N=26)	Total (N=80)
Treatment compliance based on tablets counts			
> 100%	10 (18.5)	4 (15.4)	14 (17.5)
≥ 90% to ≤ 100%	37 (68.5)	18 (69.2)	55 (68.8)
≥ 80% to < 90%	4 (7.4)	4 (15.4)	8 (10.0)
≥ 70% to < 80%	0	0	0
≥ 60% to < 70%	0	0	0
≥ 50% to < 60%	0	0	0
≥ 40% to < 50%	0	0	0
≥ 30% to < 40%	0	0	0
≥ 20% to < 30%	1 (1.9)	0	1 (1.3)
≥ 10% to < 20%	0	0	0
< 10%	0	0	0
n	52	26	78
Mean (SD)	97.0 (11.12)	97.4 (5.44)	97.1 (9.57)
Median	98.9	98.8	98.9
Min, Max	27.4, 116.0	86.3, 113.1	27.4, 116.0

% = $n/N \times 100$. Treatment duration is derived as date of last dose - date of first dose + 1.

Treatment compliance based on actual danicopan dosage is defined as the total danicopan dose amount of received divided by the total danicopan dose amount of expected to be received by patient during the period. Treatment compliance based on tablet counts is defined as the total number of tablets received divided by the total number of tablets expected to be received by patient during the period. Compliance was calculated using available study drug accountability data.

Source: ADaM.ADSL, ADaM.ADEXSUM

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Table 14.3.1.1.4
Danicopan Treatment Duration for Entire Study
Safety Set

	D->D (N=57)	P->D (N=27)	Total (N=84)
Treatment duration (days)			
n	57	27	84
Mean (SD)	441.9 (176.02)	393.7 (178.96)	426.4 (177.34)
Median	439.0	381.0	427.5
Min, Max	44.0, 769.0	77.0, 695.0	44.0, 769.0
Treatment duration (days) by dose level			
100 mg			
n	3	0	3
Mean (SD)	65.3 (49.17)		65.3 (49.17)
Median	70.0		70.0
Min, Max	14.0, 112.0		14.0, 112.0
150 mg			
n	57	27	84
Mean (SD)	157.4 (155.88)	178.0 (192.78)	164.0 (167.72)
Median	84.0	43.0	84.0
Min, Max	40.0, 669.0	41.0, 658.0	40.0, 669.0
200 mg			
n	43	16	59
Mean (SD)	371.5 (158.51)	362.9 (190.05)	369.2 (165.98)
Median	365.0	393.5	369.0
Min, Max	7.0, 666.0	30.0, 654.0	7.0, 666.0

Treatment duration is derived as date of last dose of Danicopan - date of first dose of Danicopan + 1.

For patients randomized to placebo group, only exposure duration to Danicopan was summarized.

N is number of patients who were exposed to Danicopan.

Source: ADaM.ADSL, ADaM.ADEXSUM

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