

Dossier zur Nutzenbewertung gemäß § 35a SGB V

Pegcetacoplan (Aspaveli®)

Swedish Orphan Biovitrum GmbH

Modul 4 B - Anhang 4-H

*Nicht vorbehandelte erwachsene PNH-Patienten, die
eine hämolytische Anämie haben*

Subgruppenanalysen

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PT1QEWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored) by number of transfusions previous year _____	791
PT1QTWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored) by age _____	794
PT1QTWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored) by sex _____	797
PT1QTWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored) by region _____	800

PT1QWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored) by number of transfusions previous year _____	803
PT1QSWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by age _____	806
PT1QSWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by sex _____	809
PT1QSWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by region _____	812
PT1QSWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by number of transfusions previous year _____	815
PT1QGWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by age _____	818
PT1QGWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by region _____	821
PT1QYWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored) by region _____	824
PT1QRWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by age _____	827
PT1QRWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by sex _____	830
PT1QRWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by region _____	833
PT1QRWDT10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by number of transfusions previous year _____	836
PT1QTWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored) by age _____	839
PT1QTWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored) by sex _____	842
PT1QTWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored) by region _____	845
PT1QTWDT10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored) by number of transfusions previous year _____	848
PT1QSWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by sex _____	851
PT1QSWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by region _____	854
PT1LTWC_SICC: Change from baseline in LASA Total Score (censored) by age _____	857
PT1LTWC_SIHC: Change from baseline in LASA Total Score (censored) by age _____	860
PT1LTWC_SICD: Change from baseline in LASA Total Score (censored) by sex _____	864
PT1LTWC_SIHD: Change from baseline in LASA Total Score (censored) by sex _____	867
PT1LTWC_SICB: Change from baseline in LASA Total Score (censored) by region _____	871
PT1LTWC_SIHB: Change from baseline in LASA Total Score (censored) by region _____	874
PT1LTWC_SICA: Change from baseline in LASA Total Score (censored) by number of transfusions previous year _____	878
PT1LTWC_SIHA: Change from baseline in LASA Total Score (censored) by number of transfusions previous year _____	881

PT1LAWC_SICC: Change from baseline in LASA Activity Level (censored) by age	885
PT1LAWC_SIHC: Change from baseline in LASA Activity Level (censored) by age	888
PT1LAWC_SICD: Change from baseline in LASA Activity Level (censored) by sex	892
PT1LAWC_SIHD: Change from baseline in LASA Activity Level (censored) by sex	895
PT1LAWC_SICB: Change from baseline in LASA Activity Level (censored) by region	899
PT1LAWC_SIHB: Change from baseline in LASA Activity Level (censored) by region	902
PT1LAWC_SICA: Change from baseline in LASA Activity Level (censored) by number of transfusions previous year	906
PT1LAWC_SIHA: Change from baseline in LASA Activity Level (censored) by number of transfusions previous year	909
PT1LDWC_SICC: Change from baseline in LASA Daily Activities (censored) by age	913
PT1LDWC_SIHC: Change from baseline in LASA Daily Activities (censored) by age	916
PT1LDWC_SICD: Change from baseline in LASA Daily Activities (censored) by sex	920
PT1LDWC_SIHD: Change from baseline in LASA Daily Activities (censored) by sex	923
PT1LDWC_SICB: Change from baseline in LASA Daily Activities (censored) by region	927
PT1LDWC_SIHB: Change from baseline in LASA Daily Activities (censored) by region	930
PT1LDWC_SICA: Change from baseline in LASA Daily Activities (censored) by number of transfusions previous year	934
PT1LDWC_SIHA: Change from baseline in LASA Daily Activities (censored) by number of transfusions previous year	937
PT1LQWC_SICC: Change from baseline in LASA Overall QoL (censored) by age	941
PT1LQWC_SIHC: Change from baseline in LASA Overall QoL (censored) by age	944
PT1LQWC_SICD: Change from baseline in LASA Overall QoL (censored) by sex	948
PT1LQWC_SIHD: Change from baseline in LASA Overall QoL (censored) by sex	951
PT1LQWC_SICB: Change from baseline in LASA Overall QoL (censored) by region	955
PT1LQWC_SIHB: Change from baseline in LASA Overall QoL (censored) by region	958
PT1LQWC_SICA: Change from baseline in LASA Overall QoL (censored) by number of transfusions previous year	962
PT1LQWC_SIHA: Change from baseline in LASA Overall QoL (censored) by number of transfusions previous year	965
PT1LTWIT45_SITC: Time to increase of at least 45 points in change from baseline of LASA Total Score (censored) by age	969
PT1LTWIT45_SITD: Time to increase of at least 45 points in change from baseline of LASA Total Score (censored) by sex	972
PT1LTWIT45_SITA: Time to increase of at least 45 points in change from baseline of LASA Total Score (censored) by number of transfusions previous year	975
PT1LAWIT15_SITC: Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored) by age	978
PT1LAWIT15_SITD: Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored) by sex	981
PT1LAWIT15_SITB: Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored) by region	984

PT1LAWIT15_SITA: Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored) by number of transfusions previous year	___	987
PT1LDWIT15_SITC: Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by age	_____	990
PT1LDWIT15_SITD: Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by sex	_____	993
PT1LDWIT15_SITB: Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by region	_____	996
PT1LDWIT15_SITA: Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by number of transfusions previous year	_____	999
PT1LQWIT15_SITC: Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored) by age	_____	1002
PT1LQWIT15_SITD: Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored) by sex	_____	1005
PT1LQWIT15_SITB: Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored) by region	_____	1008
PT1LQWIT15_SITA: Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored) by number of transfusions previous year	___	1011
PT1LAWDT15_SITD: Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored) by sex	_____	1014
PT1LAWDT15_SITB: Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored) by region	_____	1017
PT1LAWDT15_SITA: Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored) by number of transfusions previous year	___	1020
PT1LDWDT15_SITB: Time to decrease of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by region	_____	1023
PT1LDWDT15_SITA: Time to decrease of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by number of transfusions previous year	_____	1026
PT1LQWDT15_SITB: Time to decrease of at least 15 points in change from baseline of LASA Overall QoL (censored) by region	_____	1029
PT1A_SSNC: TEAEs during RCP by age	_____	1032
PT1A_SSND: TEAEs during RCP by sex	_____	1035
PT1A_SSNB: TEAEs during RCP by region	_____	1038
PT1A_SSNA: TEAEs during RCP by number of transfusions previous year	_____	1041
PT1AM_SSNC: Mild TEAEs during RCP by age	_____	1044
PT1AM_SSND: Mild TEAEs during RCP by sex	_____	1047
PT1AM_SSNB: Mild TEAEs during RCP by region	_____	1050
PT1AM_SSNA: Mild TEAEs during RCP by number of transfusions previous year	_____	1053
PT1AI_SSNC: Moderate TEAEs during RCP by age	_____	1056
PT1AI_SSND: Moderate TEAEs during RCP by sex	_____	1059
PT1AI_SSNB: Moderate TEAEs during RCP by region	_____	1062
PT1AI_SSNA: Moderate TEAEs during RCP by number of transfusions previous year	_____	1065
PT1AA_SSNC: Non-disease related TEAEs during RCP by age	_____	1068

PT1AA_SSND: Non-disease related TEAEs during RCP by sex	1071
PT1AA_SSNB: Non-disease related TEAEs during RCP by region	1074
PT1AA_SSNA: Non-disease related TEAEs during RCP by number of transfusions previous year	1077
PT1AF_SSNC: AESI Infections and Infestations during RCP by age	1080
PT1AL_SSNC: AESI Injection site reaction during RCP by age	1083
PT1ALM_SSNC: Mild AESI Injection site reaction during RCP by age	1085

Table PT1HBS_SIPC: Hb stabilisation during RCP by age
 ITT

Variable	Subgroup	Time	Peg		SoC		RR	OR	RD	p-value IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]				
Hb stabilisation during RCP	Age	RCP								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 Hb stabilization: avoidance of a > 1 g/dL decrease in Hb levels from baseline in the absence of transfusions.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1HBS_SIPC: Hb stabilisation during RCP by age
 ITT

Variable	Subgroup	Time	Peg		SoC		RR [95 % CI]	OR [95 % CI]	RD [95 % CI]	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb stabilisation during RCP	< 60	RCP	29	24 (82.8) [64.2, 94.2]	13	0 (0.0) [NE, NE]	NE [NE, NE]	NE [NE, NE]	82.8 [54.6, 94.6]	<0.0001	*

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 Hb stabilization: avoidance of a > 1 g/dL decrease in Hb levels from baseline in the absence of transfusions.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1HBS_SIPC: Hb stabilisation during RCP by age
 ITT

Variable	Subgroup	Time	Peg		SoC		RR [95 % CI]	OR [95 % CI]	RD [95 % CI]	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb stabilisation during RCP	>= 60	RCP	6	6 (100.0) [54.1, 100.0]	5	0 (0.0) [NE, NE]	NE [NE, NE]	NE [NE, NE]	100.0 [42.4, 100.0]	0.0022	*

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 Hb stabilization: avoidance of a > 1 g/dL decrease in Hb levels from baseline in the absence of transfusions.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1HBS_SIPD: Hb stabilisation during RCP by sex
 ITT

Variable	Subgroup	Time	Peg		SoC		RR	OR	RD	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb stabilisation during RCP	Sex	RCP									NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 Hb stabilization: avoidance of a > 1 g/dL decrease in Hb levels from baseline in the absence of transfusions.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1HBS_SIPD: Hb stabilisation during RCP by sex
 ITT

Variable	Subgroup	Time	Peg		SoC		RR [95 % CI]	OR [95 % CI]	RD [95 % CI]	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb stabilisation during RCP	Male	RCP	19	15 (78.9) [54.4, 93.9]	10	0 (0.0) [NE, NE]	NE [NE, NE]	NE [NE, NE]	78.9 [44.4, 93.9]	<0.0001	*

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 Hb stabilization: avoidance of a > 1 g/dL decrease in Hb levels from baseline in the absence of transfusions.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1HBS_SIPD: Hb stabilisation during RCP by sex
 ITT

Variable	Subgroup	Time	Peg		SoC		RR [95 % CI]	OR [95 % CI]	RD [95 % CI]	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb stabilisation during RCP	Female	RCP	16	15 (93.8) [69.8, 99.8]	8	0 (0.0) [NE, NE]	NE [NE, NE]	NE [NE, NE]	93.8 [58.5, 99.8]	<0.0001	*

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 Hb stabilization: avoidance of a > 1 g/dL decrease in Hb levels from baseline in the absence of transfusions.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1HBS_SIPB: Hb stabilisation during RCP by region
 ITT

Variable	Subgroup	Time	Peg		SoC		RR	OR	RD	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb stabilisation during RCP	Region	RCP									NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 Hb stabilization: avoidance of a > 1 g/dL decrease in Hb levels from baseline in the absence of transfusions.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1HBS_SIPB: Hb stabilisation during RCP by region
 ITT

Variable	Subgroup	Time	Peg		SoC		RR [95 % CI]	OR [95 % CI]	RD [95 % CI]	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb stabilisation during RCP	Latin America	RCP	12	8 (66.7) [34.9, 90.1]	2	0 (0.0) [NE, NE]	NE [NE, NE]	NE [NE, NE]	66.7 [-19.6, 90.1]	0.1648	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 Hb stabilization: avoidance of a > 1 g/dL decrease in Hb levels from baseline in the absence of transfusions.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1HBS_SIPB: Hb stabilisation during RCP by region
 ITT

Variable	Subgroup	Time	Peg		SoC		RR [95 % CI]	OR [95 % CI]	RD [95 % CI]	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb stabilisation during RCP	Asia Pacific	RCP	23	22 (95.7) [78.1, 99.9]	16	0 (0.0) [NE, NE]	NE [NE, NE]	NE [NE, NE]	95.7 [75.3, 99.9]	<0.0001	*

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 Hb stabilization: avoidance of a > 1 g/dL decrease in Hb levels from baseline in the absence of transfusions.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1HBS_SIPA: Hb stabilisation during RCP by number of transfusions previous year
 ITT

Variable	Subgroup	Time	Peg		SoC		RR	OR	RD	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb stabilisation during RCP	Number of transfusions in the previous year	RCP									NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 Hb stabilization: avoidance of a > 1 g/dL decrease in Hb levels from baseline in the absence of transfusions.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1HBS_SIPA: Hb stabilisation during RCP by number of transfusions previous year
 ITT

Variable	Subgroup	Time	Peg		SoC		RR [95 % CI]	OR [95 % CI]	RD [95 % CI]	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb stabilisation during RCP	< 4	RCP	21	18 (85.7) [63.7, 97.0]	8	0 (0.0) [NE, NE]	NE [NE, NE]	NE [NE, NE]	85.7 [47.3, 97.0]	<0.0001	*

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 Hb stabilization: avoidance of a > 1 g/dL decrease in Hb levels from baseline in the absence of transfusions.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1HBS_SIPA: Hb stabilisation during RCP by number of transfusions previous year
 ITT

Variable	Subgroup	Time	Peg		SoC		RR [95 % CI]	OR [95 % CI]	RD [95 % CI]	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb stabilisation during RCP	>= 4	RCP	14	12 (85.7) [57.2, 98.2]	10	0 (0.0) [NE, NE]	NE [NE, NE]	NE [NE, NE]	85.7 [52.1, 98.2]	<0.0001	*

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 Hb stabilization: avoidance of a > 1 g/dL decrease in Hb levels from baseline in the absence of transfusions.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1HBWC_SICC: Change from baseline in Hb (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA				
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA	
Age	Peg	Overall									0.7750

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Hb = Hemoglobin levels (g/dl).
 Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SICC: Change from baseline in Hb (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 60	Peg	Week 2	29	28 (96.6)	1.86 (0.26)	(1.33, 2.39)	1.87 (0.49)	(0.89, 2.86)	0.0005	*
		Week 4	29	27 (93.1)	2.33 (0.29)	(1.75, 2.91)	3.07 (0.52)	(2.02, 4.12)	<0.0001	*
		Week 6	29	27 (93.1)	2.61 (0.27)	(2.06, 3.17)	2.78 (0.49)	(1.79, 3.77)	<0.0001	*
		Week 8	29	27 (93.1)	2.80 (0.37)	(2.05, 3.55)	3.07 (0.67)	(1.72, 4.42)	<0.0001	*
		Week 10	29	26 (89.7)	2.82 (0.41)	(1.99, 3.64)	2.70 (0.72)	(1.24, 4.16)	0.0006	*
		Week 12	29	28 (96.6)	2.58 (0.35)	(1.87, 3.29)	2.28 (0.64)	(0.99, 3.58)	0.0010	*
		Week 14	29	28 (96.6)	2.48 (0.34)	(1.79, 3.17)	2.55 (0.62)	(1.28, 3.81)	0.0002	*
		Week 16	29	28 (96.6)	2.68 (0.39)	(1.88, 3.47)	2.99 (0.72)	(1.53, 4.44)	0.0002	*
		Week 18	29	28 (96.6)	2.88 (0.38)	(2.11, 3.64)	2.63 (0.69)	(1.24, 4.03)	0.0005	*
		Week 20	29	27 (93.1)	3.22 (0.36)	(2.49, 3.94)	3.25 (0.66)	(1.90, 4.59)	<0.0001	*
		Week 22	29	28 (96.6)	3.26 (0.34)	(2.58, 3.94)	3.01 (0.63)	(1.73, 4.29)	<0.0001	*
		Week 24	29	28 (96.6)	3.17 (0.40)	(2.36, 3.98)	3.01 (0.73)	(1.53, 4.49)	0.0002	*
		Week 26	29	24 (82.8)	3.18 (0.43)	(2.31, 4.06)	2.99 (0.78)	(1.40, 4.58)	0.0006	*
		SoC	Week 2	13	12 (92.3)	-0.01 (0.40)	(-0.83, 0.80)			
	Week 4		13	13 (100.0)	-0.74 (0.42)	(-1.60, 0.11)				
	Week 6		13	13 (100.0)	-0.17 (0.40)	(-0.97, 0.63)				
	Week 8		13	13 (100.0)	-0.27 (0.54)	(-1.37, 0.82)				
	Week 10		13	13 (100.0)	0.12 (0.58)	(-1.06, 1.30)				
	Week 12		13	13 (100.0)	0.30 (0.52)	(-0.76, 1.36)				
	Week 14		13	13 (100.0)	-0.06 (0.51)	(-1.09, 0.97)				
	Week 16		13	13 (100.0)	-0.31 (0.58)	(-1.49, 0.87)				
	Week 18		13	13 (100.0)	0.25 (0.56)	(-0.89, 1.38)				
	Week 20		13	12 (92.3)	-0.03 (0.54)	(-1.13, 1.07)				
	Week 22		13	12 (92.3)	0.25 (0.52)	(-0.80, 1.30)				
	Week 24		13	13 (100.0)	0.16 (0.60)	(-1.04, 1.37)				
	Week 26	13	12 (92.3)	0.19 (0.62)	(-1.08, 1.46)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.

ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.

ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to

Peg. NE = not evaluable. IA = p-value of interaction term.

Hb = Hemoglobin levels (g/dl).

Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SICC: Change from baseline in Hb (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 2	6	5 (83.3)	1.81 (0.70)	(0.08, 3.53)	2.44 (1.01)	(-0.03, 4.91)	0.0520	
		Week 4	6	6 (100.0)	2.40 (0.75)	(0.57, 4.23)	3.36 (1.17)	(0.49, 6.22)	0.0285	*
		Week 6	6	6 (100.0)	2.97 (0.71)	(1.29, 4.66)	2.75 (1.08)	(0.21, 5.30)	0.0377	*
		Week 8	6	6 (100.0)	2.65 (0.55)	(1.35, 3.95)	3.94 (0.83)	(1.98, 5.91)	0.0021	*
		Week 10	6	6 (100.0)	2.96 (0.76)	(1.17, 4.76)	3.37 (1.15)	(0.66, 6.08)	0.0218	*
		Week 12	6	6 (100.0)	2.20 (0.93)	(0.00, 4.39)	2.20 (1.40)	(-1.12, 5.51)	0.1608	
		Week 14	6	6 (100.0)	2.57 (0.83)	(0.61, 4.52)	3.27 (1.25)	(0.31, 6.23)	0.0348	*
		Week 16	6	6 (100.0)	2.70 (0.80)	(0.80, 4.59)	3.21 (1.21)	(0.36, 6.07)	0.0325	*
		Week 18	6	6 (100.0)	2.70 (0.75)	(0.93, 4.48)	2.72 (1.13)	(0.04, 5.40)	0.0472	*
		Week 20	6	6 (100.0)	2.75 (0.76)	(0.94, 4.55)	3.26 (1.15)	(0.53, 5.99)	0.0256	*
		Week 22	6	5 (83.3)	3.01 (0.77)	(1.14, 4.89)	3.26 (1.10)	(0.56, 5.96)	0.0253	*
		Week 24	6	6 (100.0)	3.17 (0.80)	(1.28, 5.07)	4.00 (1.21)	(1.14, 6.87)	0.0130	*
		Week 26	6	6 (100.0)	2.96 (0.79)	(0.92, 5.00)	3.84 (1.40)	(0.25, 7.42)	0.0404	*
		SoC	Week 2	5	5 (100.0)	-0.64 (0.70)	(-2.36, 1.09)			
	Week 4		5	4 (80.0)	-0.96 (0.89)	(-3.13, 1.20)				
	Week 6		5	5 (100.0)	0.22 (0.76)	(-1.59, 2.03)				
	Week 8		5	5 (100.0)	-1.29 (0.59)	(-2.69, 0.10)				
	Week 10		5	5 (100.0)	-0.40 (0.81)	(-2.33, 1.52)				
	Week 12		5	5 (100.0)	-0.00 (1.00)	(-2.36, 2.35)				
	Week 14		5	5 (100.0)	-0.70 (0.89)	(-2.80, 1.40)				
	Week 16		5	5 (100.0)	-0.52 (0.86)	(-2.55, 1.51)				
	Week 18		5	5 (100.0)	-0.02 (0.80)	(-1.92, 1.88)				
	Week 20		5	5 (100.0)	-0.51 (0.82)	(-2.45, 1.42)				
	Week 22		5	5 (100.0)	-0.24 (0.77)	(-2.12, 1.63)				
	Week 24		5	5 (100.0)	-0.83 (0.86)	(-2.87, 1.20)				
	Week 26	5	3 (60.0)	-0.88 (1.10)	(-3.71, 1.96)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.

ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.

ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to

Peg. NE = not evaluable. IA = p-value of interaction term.

Hb = Hemoglobin levels (g/dl).

Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SIHC: Change from baseline in Hb (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Hb level (censored)	< 60	Baseline	Peg	29	29 (100.0)	9.40 (1.38)	6.7	9.15	13.1	
			SoC	13	13 (100.0)	8.63 (0.83)	7.2	8.68	10.0	
		Week 2	Peg	29	28 (96.6)	11.27 (1.69)	7.8	11.40	14.4	
			SoC	13	12 (92.3)	8.73 (1.69)	6.4	8.75	11.9	
		Week 4	Peg	29	27 (93.1)	11.74 (1.97)	7.5	11.90	16.0	
			SoC	13	13 (100.0)	8.05 (1.36)	6.5	7.90	10.7	
		Week 6	Peg	29	27 (93.1)	12.07 (1.84)	7.8	12.10	15.6	
			SoC	13	13 (100.0)	8.55 (1.71)	5.9	8.10	11.4	
		Week 8	Peg	29	27 (93.1)	12.11 (2.33)	4.4	12.44	15.6	
			SoC	13	13 (100.0)	8.39 (1.83)	5.4	8.50	11.4	
		Week 10	Peg	29	26 (89.7)	12.13 (2.38)	5.1	12.70	15.7	
			SoC	13	13 (100.0)	8.81 (1.96)	5.4	8.50	11.8	
		Week 12	Peg	29	28 (96.6)	12.06 (2.22)	6.7	12.40	15.1	
			SoC	13	13 (100.0)	8.98 (2.34)	5.4	8.40	14.0	
		Week 14	Peg	29	28 (96.6)	11.96 (2.42)	5.4	12.45	16.6	
			SoC	13	13 (100.0)	8.60 (1.65)	5.4	8.30	11.4	
		Week 16	Peg	29	28 (96.6)	12.15 (2.71)	5.4	12.70	16.1	
			SoC	13	13 (100.0)	8.32 (1.55)	5.4	7.90	11.4	
		Week 18	Peg	29	28 (96.6)	12.31 (2.38)	4.4	12.75	16.0	
			SoC	13	13 (100.0)	8.96 (1.86)	5.4	8.60	11.5	
		Week 20	Peg	29	27 (93.1)	12.63 (1.99)	8.3	12.80	16.2	
			SoC	13	12 (92.3)	8.78 (2.00)	5.4	8.55	11.4	
		Week 22	Peg	29	28 (96.6)	12.71 (2.14)	8.6	12.80	17.1	
			SoC	13	12 (92.3)	8.93 (2.04)	5.4	8.65	11.4	
		Week 24	Peg	29	28 (96.6)	12.62 (2.46)	7.1	13.05	16.5	
			SoC	13	13 (100.0)	8.91 (2.20)	5.4	8.60	13.0	
		Week 26	Peg	29	24 (82.8)	12.84 (2.29)	8.0	13.05	16.2	
			SoC	13	12 (92.3)	9.00 (2.45)	5.4	8.95	14.4	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Hb = Hemoglobin levels (g/dl).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SIHC: Change from baseline in Hb (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Hb level (censored)	>= 60	Baseline	Peg	6	6 (100.0)	9.34 (1.63)	6.5	9.63	11.3	
			SoC	5	5 (100.0)	8.80 (0.68)	8.0	8.50	9.7	
	Week 2	Peg	6	5 (83.3)	10.71 (0.73)	9.9	10.60	11.6		
		SoC	5	5 (100.0)	8.22 (1.78)	5.9	8.00	10.4		
	Week 4	Peg	6	6 (100.0)	11.65 (1.23)	9.7	11.70	13.1		
		SoC	5	4 (80.0)	8.16 (1.96)	6.6	7.60	10.8		
	Week 6	Peg	6	6 (100.0)	12.33 (1.72)	10.5	12.05	14.6		
		SoC	5	5 (100.0)	9.13 (1.52)	7.6	8.62	11.1		
	Week 8	Peg	6	6 (100.0)	12.04 (1.81)	9.6	12.50	14.0		
		SoC	5	5 (100.0)	7.64 (1.10)	6.1	7.90	9.1		
	Week 10	Peg	6	6 (100.0)	12.35 (1.86)	10.2	12.60	14.7		
		SoC	5	5 (100.0)	8.49 (1.67)	6.1	8.70	10.6		
	Week 12	Peg	6	6 (100.0)	11.64 (2.36)	9.2	11.15	14.6		
		SoC	5	5 (100.0)	8.87 (1.92)	6.1	9.11	10.6		
	Week 14	Peg	6	6 (100.0)	11.85 (1.94)	9.7	11.90	14.1		
		SoC	5	5 (100.0)	8.29 (1.70)	6.1	7.90	10.6		
	Week 16	Peg	6	6 (100.0)	12.01 (2.06)	9.1	12.44	14.6		
		SoC	5	5 (100.0)	8.47 (1.67)	6.1	8.60	10.6		
	Week 18	Peg	6	6 (100.0)	12.10 (1.76)	9.6	12.39	14.0		
		SoC	5	5 (100.0)	8.90 (1.96)	6.1	9.11	10.8		
	Week 20	Peg	6	6 (100.0)	12.04 (1.89)	9.7	12.30	14.5		
		SoC	5	5 (100.0)	8.49 (1.67)	6.1	8.70	10.6		
	Week 22	Peg	6	5 (83.3)	11.95 (1.75)	9.9	12.20	14.5		
		SoC	5	5 (100.0)	8.57 (1.69)	6.1	9.10	10.6		
	Week 24	Peg	6	6 (100.0)	12.37 (1.78)	10.2	12.30	15.0		
		SoC	5	5 (100.0)	8.23 (1.73)	6.1	7.90	10.6		
	Week 26	Peg	6	6 (100.0)	12.43 (1.39)	10.9	12.20	14.4		
		SoC	5	3 (60.0)	8.22 (2.29)	6.1	7.90	10.6		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Hb = Hemoglobin levels (g/dl).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SIHC: Change from baseline in Hb (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in Hb (censored)	Week 2	Peg	29	28 (96.6)	1.84 (1.28)	-0.9	2.02	4.0	1.21 (0.38) [0.53, 2.03]
		SoC	13	12 (92.3)	0.10 (1.70)	-2.3	-0.16	3.8	
	Week 4	Peg	29	27 (93.1)	2.29 (1.49)	-0.7	2.29	4.6	1.81 (0.41) [1.11, 2.72]
		SoC	13	13 (100.0)	-0.58 (1.70)	-2.1	-0.96	3.4	
	Week 6	Peg	29	27 (93.1)	2.64 (1.43)	-1.2	2.95	5.1	1.85 (0.41) [1.14, 2.76]
		SoC	13	13 (100.0)	-0.08 (1.48)	-2.6	-0.49	2.4	
	Week 8	Peg	29	27 (93.1)	2.82 (1.97)	-4.6	3.29	5.4	1.57 (0.40) [0.88, 2.43]
		SoC	13	13 (100.0)	-0.24 (1.81)	-3.1	-0.44	2.9	
	Week 10	Peg	29	26 (89.7)	2.84 (2.05)	-3.8	3.26	6.5	1.28 (0.38) [0.60, 2.11]
		SoC	13	13 (100.0)	0.18 (2.00)	-3.1	0.33	3.6	
	Week 12	Peg	29	28 (96.6)	2.64 (1.81)	-2.3	2.83	6.6	1.17 (0.37) [0.51, 1.97]
		SoC	13	13 (100.0)	0.35 (2.15)	-3.1	0.33	5.5	
	Week 14	Peg	29	28 (96.6)	2.53 (1.90)	-3.6	3.08	5.0	1.37 (0.38) [0.70, 2.19]
		SoC	13	13 (100.0)	-0.03 (1.70)	-3.1	-0.45	3.4	
	Week 16	Peg	29	28 (96.6)	2.72 (2.29)	-3.8	3.32	6.2	1.46 (0.39) [0.78, 2.30]
		SoC	13	13 (100.0)	-0.31 (1.29)	-3.1	-0.49	1.9	
	Week 18	Peg	29	28 (96.6)	2.88 (2.03)	-4.6	3.20	6.0	1.28 (0.38) [0.61, 2.10]
		SoC	13	13 (100.0)	0.33 (1.78)	-3.1	0.33	3.5	
	Week 20	Peg	29	27 (93.1)	3.18 (1.60)	-0.3	3.30	6.2	1.61 (0.41) [0.90, 2.50]
		SoC	13	12 (92.3)	0.14 (2.34)	-3.1	-0.08	3.7	
	Week 22	Peg	29	28 (96.6)	3.29 (1.66)	0.0	3.78	6.3	1.62 (0.40) [0.91, 2.50]
		SoC	13	12 (92.3)	0.29 (2.14)	-3.1	0.65	3.8	
	Week 24	Peg	29	28 (96.6)	3.19 (2.09)	-1.9	3.40	7.1	1.32 (0.38) [0.65, 2.14]
		SoC	13	13 (100.0)	0.28 (2.29)	-3.1	0.33	5.6	
	Week 26	Peg	29	24 (82.8)	3.18 (1.82)	-0.6	3.47	6.8	1.28 (0.40) [0.58, 2.16]
		SoC	13	12 (92.3)	0.34 (2.74)	-3.1	0.38	6.9	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Hb = Hemoglobin levels (g/dl).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SIHC: Change from baseline in Hb (censored) by age
ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in Hb (censored)	Week 2	Peg	6	5 (83.3)	1.75 (1.83)	-0.0	1.25	4.8	1.13 (0.82) [-0.11, 3.12]
		SoC	5	5 (100.0)	-0.58 (1.88)	-2.6	-0.50	1.4	
	Week 4	Peg	6	6 (100.0)	2.31 (2.30)	-0.2	1.94	6.6	1.38 (0.87) [0.11, 3.53]
		SoC	5	4 (80.0)	-0.71 (1.29)	-1.9	-1.06	1.1	
	Week 6	Peg	6	6 (100.0)	2.99 (2.35)	0.6	2.51	7.3	1.25 (0.78) [0.06, 3.14]
		SoC	5	5 (100.0)	0.33 (1.29)	-1.3	0.11	1.8	
	Week 8	Peg	6	6 (100.0)	2.70 (2.54)	-0.3	2.66	7.0	1.65 (0.85) [0.46, 3.76]
		SoC	5	5 (100.0)	-1.16 (1.48)	-2.5	-1.39	1.1	
	Week 10	Peg	6	6 (100.0)	3.01 (2.50)	0.3	3.06	7.3	1.43 (0.81) [0.24, 3.41]
		SoC	5	5 (100.0)	-0.31 (1.53)	-2.4	0.25	1.1	
	Week 12	Peg	6	6 (100.0)	2.30 (2.91)	-0.1	1.51	7.5	0.81 (0.73) [-0.40, 2.49]
		SoC	5	5 (100.0)	0.07 (1.89)	-2.4	0.94	2.1	
	Week 14	Peg	6	6 (100.0)	2.51 (2.83)	-0.1	1.83	7.6	1.18 (0.77) [-0.01, 3.03]
		SoC	5	5 (100.0)	-0.51 (1.50)	-2.4	-0.75	1.1	
	Week 16	Peg	6	6 (100.0)	2.67 (3.03)	-0.8	2.47	8.1	1.11 (0.76) [-0.08, 2.92]
		SoC	5	5 (100.0)	-0.33 (1.52)	-2.4	0.15	1.1	
	Week 18	Peg	6	6 (100.0)	2.76 (2.64)	-0.3	2.58	7.5	1.03 (0.75) [-0.17, 2.80]
		SoC	5	5 (100.0)	0.11 (1.94)	-2.4	0.94	2.3	
	Week 20	Peg	6	6 (100.0)	2.70 (2.91)	-0.2	2.23	8.0	1.15 (0.77) [-0.04, 2.98]
		SoC	5	5 (100.0)	-0.31 (1.53)	-2.4	0.25	1.1	
	Week 22	Peg	6	5 (83.3)	3.00 (3.07)	-0.0	2.19	8.0	1.19 (0.83) [-0.05, 3.21]
		SoC	5	5 (100.0)	-0.23 (1.57)	-2.4	0.65	1.1	
	Week 24	Peg	6	6 (100.0)	3.03 (2.86)	0.3	2.49	8.5	1.39 (0.80) [0.21, 3.36]
		SoC	5	5 (100.0)	-0.57 (1.52)	-2.4	-1.05	1.1	
	Week 26	Peg	6	6 (100.0)	3.09 (2.46)	1.0	2.49	7.9	1.58 (1.02) [0.20, 4.16]
		SoC	5	3 (60.0)	-0.95 (1.72)	-2.4	-1.39	0.9	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
Hb = Hemoglobin levels (g/dl).
Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SICD: Change from baseline in Hb (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.7850

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Hb = Hemoglobin levels (g/dl).
 Source Data: aeфф, created on: 13SEP2023

Table PT1HBWC_SICD: Change from baseline in Hb (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA	
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value		
Male	Peg	Week 2	19	18 (94.7)	1.67 (0.35)	(0.94, 2.40)	2.45 (0.64)	(1.13, 3.76)	0.0008	*	
		Week 4	19	18 (94.7)	2.21 (0.42)	(1.35, 3.07)	3.19 (0.74)	(1.66, 4.71)	0.0002	*	
		Week 6	19	19 (100.0)	2.55 (0.39)	(1.74, 3.35)	2.65 (0.70)	(1.21, 4.09)	0.0009	*	
		Week 8	19	18 (94.7)	2.38 (0.47)	(1.41, 3.36)	3.55 (0.83)	(1.84, 5.25)	0.0002	*	
		Week 10	19	17 (89.5)	2.48 (0.57)	(1.31, 3.66)	2.42 (0.97)	(0.41, 4.43)	0.0202	*	
		Week 12	19	19 (100.0)	2.37 (0.46)	(1.43, 3.31)	2.12 (0.82)	(0.43, 3.80)	0.0157	*	
		Week 14	19	19 (100.0)	2.23 (0.46)	(1.29, 3.18)	2.75 (0.82)	(1.06, 4.44)	0.0025	*	
		Week 16	19	19 (100.0)	2.52 (0.54)	(1.40, 3.63)	3.27 (0.97)	(1.27, 5.27)	0.0025	*	
		Week 18	19	19 (100.0)	2.55 (0.52)	(1.48, 3.62)	2.29 (0.93)	(0.37, 4.20)	0.0213	*	
		Week 20	19	19 (100.0)	3.09 (0.45)	(2.16, 4.02)	3.36 (0.83)	(1.65, 5.07)	0.0005	*	
		Week 22	19	18 (94.7)	3.34 (0.48)	(2.35, 4.32)	3.09 (0.86)	(1.31, 4.86)	0.0016	*	
		Week 24	19	19 (100.0)	3.20 (0.57)	(2.03, 4.38)	3.26 (1.02)	(1.16, 5.36)	0.0038	*	
		Week 26	19	17 (89.5)	3.60 (0.53)	(2.50, 4.70)	3.63 (0.94)	(1.68, 5.58)	0.0009	*	
		SoC	SoC	Week 2	10	9 (90.0)	-0.78 (0.52)	(-1.85, 0.30)			
	Week 4			10	10 (100.0)	-0.98 (0.59)	(-2.20, 0.25)				
	Week 6			10	10 (100.0)	-0.10 (0.57)	(-1.28, 1.07)				
	Week 8			10	10 (100.0)	-1.16 (0.66)	(-2.53, 0.21)				
	Week 10			10	10 (100.0)	0.06 (0.78)	(-1.55, 1.67)				
	Week 12			10	10 (100.0)	0.25 (0.66)	(-1.11, 1.62)				
	Week 14			10	10 (100.0)	-0.52 (0.67)	(-1.89, 0.86)				
	Week 16			10	10 (100.0)	-0.76 (0.79)	(-2.38, 0.87)				
	Week 18			10	10 (100.0)	0.26 (0.76)	(-1.30, 1.82)				
	Week 20			10	9 (90.0)	-0.27 (0.68)	(-1.68, 1.14)				
	Week 22			10	9 (90.0)	0.25 (0.70)	(-1.20, 1.70)				
	Week 24			10	10 (100.0)	-0.06 (0.83)	(-1.77, 1.66)				
	Week 26	10	9 (90.0)	-0.03 (0.76)	(-1.61, 1.55)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.

ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.

ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to

Peg. NE = not evaluable. IA = p-value of interaction term.

Hb = Hemoglobin levels (g/dl).

Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SICD: Change from baseline in Hb (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	Treatment Difference		p-value	IA
Female	Peg	Week 2	16	15 (93.8)	2.08 (0.34)	(1.37, 2.80)	1.84 (0.64)	(0.49, 3.18)	0.0099	*
		Week 4	16	15 (93.8)	2.55 (0.31)	(1.89, 3.20)	3.56 (0.60)	(2.30, 4.83)	<0.0001	*
		Week 6	16	14 (87.5)	2.91 (0.31)	(2.25, 3.57)	3.18 (0.58)	(1.97, 4.40)	<0.0001	*
		Week 8	16	15 (93.8)	3.31 (0.42)	(2.43, 4.18)	3.73 (0.79)	(2.08, 5.38)	0.0001	*
		Week 10	16	15 (93.8)	3.29 (0.42)	(2.41, 4.16)	3.69 (0.78)	(2.05, 5.33)	0.0002	*
		Week 12	16	15 (93.8)	2.78 (0.47)	(1.81, 3.76)	3.27 (0.88)	(1.44, 5.10)	0.0014	*
		Week 14	16	15 (93.8)	2.87 (0.42)	(1.98, 3.75)	3.42 (0.79)	(1.76, 5.07)	0.0004	*
		Week 16	16	15 (93.8)	2.95 (0.45)	(2.01, 3.89)	3.46 (0.84)	(1.70, 5.22)	0.0006	*
		Week 18	16	15 (93.8)	3.31 (0.40)	(2.47, 4.14)	3.83 (0.75)	(2.25, 5.40)	<0.0001	*
		Week 20	16	14 (87.5)	3.30 (0.48)	(2.30, 4.31)	3.91 (0.89)	(2.05, 5.76)	0.0003	*
		Week 22	16	15 (93.8)	3.20 (0.40)	(2.37, 4.03)	3.76 (0.74)	(2.21, 5.32)	<0.0001	*
		Week 24	16	15 (93.8)	3.18 (0.40)	(2.34, 4.02)	3.80 (0.75)	(2.23, 5.38)	<0.0001	*
		Week 26	16	13 (81.3)	2.69 (0.47)	(1.68, 3.69)	3.60 (1.00)	(1.48, 5.73)	0.0026	*
		SoC	Week 2	8	8 (100.0)	0.25 (0.51)	(-0.83, 1.32)			
	Week 4		8	7 (87.5)	-1.01 (0.49)	(-2.05, 0.02)				
	Week 6		8	8 (100.0)	-0.27 (0.45)	(-1.22, 0.68)				
	Week 8		8	8 (100.0)	-0.42 (0.63)	(-1.75, 0.90)				
	Week 10		8	8 (100.0)	-0.40 (0.63)	(-1.72, 0.91)				
	Week 12		8	8 (100.0)	-0.49 (0.70)	(-1.96, 0.98)				
	Week 14		8	8 (100.0)	-0.55 (0.63)	(-1.88, 0.77)				
	Week 16		8	8 (100.0)	-0.51 (0.68)	(-1.93, 0.90)				
	Week 18		8	8 (100.0)	-0.52 (0.60)	(-1.78, 0.74)				
	Week 20		8	8 (100.0)	-0.60 (0.69)	(-2.05, 0.85)				
	Week 22		8	8 (100.0)	-0.56 (0.60)	(-1.81, 0.68)				
	Week 24		8	8 (100.0)	-0.62 (0.60)	(-1.88, 0.65)				
	Week 26	8	6 (75.0)	-0.92 (0.82)	(-2.66, 0.83)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.

ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.

ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to

Peg. NE = not evaluable. IA = p-value of interaction term.

Hb = Hemoglobin levels (g/dl).

Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SIHD: Change from baseline in Hb (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Hb level (censored)	Male	Baseline	Peg	19	19 (100.0)	9.71 (1.48)	6.5	9.35	13.1	
			SoC	10	10 (100.0)	8.75 (0.86)	7.2	8.87	9.8	
		Week 2	Peg	19	18 (94.7)	11.22 (1.78)	7.8	11.35	14.4	
			SoC	10	9 (90.0)	8.50 (1.60)	5.9	8.60	11.0	
		Week 4	Peg	19	18 (94.7)	11.84 (2.10)	7.9	11.95	16.0	
			SoC	10	10 (100.0)	8.41 (1.68)	6.6	7.90	10.8	
		Week 6	Peg	19	19 (100.0)	12.23 (2.11)	7.8	12.33	15.6	
			SoC	10	10 (100.0)	9.16 (1.58)	7.3	8.85	11.2	
		Week 8	Peg	19	18 (94.7)	11.89 (2.63)	4.4	12.60	15.6	
			SoC	10	10 (100.0)	8.26 (1.45)	6.4	7.85	10.7	
		Week 10	Peg	19	17 (89.5)	12.04 (2.77)	5.1	12.83	15.7	
			SoC	10	10 (100.0)	9.24 (1.74)	6.4	8.90	11.8	
		Week 12	Peg	19	19 (100.0)	12.12 (2.47)	6.7	12.40	15.1	
			SoC	10	10 (100.0)	9.70 (2.16)	7.3	9.49	14.0	
		Week 14	Peg	19	19 (100.0)	11.98 (2.75)	5.4	12.80	16.6	
			SoC	10	10 (100.0)	8.90 (1.33)	7.7	8.20	10.8	
		Week 16	Peg	19	19 (100.0)	12.24 (3.04)	5.4	13.30	16.1	
			SoC	10	10 (100.0)	8.63 (1.24)	7.0	8.25	10.7	
		Week 18	Peg	19	19 (100.0)	12.27 (2.83)	4.4	13.00	16.0	
			SoC	10	10 (100.0)	9.53 (1.59)	7.1	10.07	11.5	
		Week 20	Peg	19	19 (100.0)	12.71 (2.29)	8.3	13.30	16.2	
			SoC	10	9 (90.0)	9.17 (1.46)	7.7	8.70	10.9	
		Week 22	Peg	19	18 (94.7)	12.91 (2.53)	8.6	13.25	17.1	
			SoC	10	9 (90.0)	9.60 (1.46)	7.7	10.02	11.4	
		Week 24	Peg	19	19 (100.0)	12.85 (2.86)	7.1	13.50	16.5	
			SoC	10	10 (100.0)	9.42 (1.90)	7.4	9.05	13.0	
		Week 26	Peg	19	17 (89.5)	13.31 (2.44)	8.0	13.60	16.2	
			SoC	10	9 (90.0)	9.59 (2.17)	7.3	9.00	14.4	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Hb = Hemoglobin levels (g/dl).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeFF, created on: 13SEP2023

Table PT1HBWC_SIHD: Change from baseline in Hb (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Hb level (censored)	Female	Baseline	Peg	16	16 (100.0)	9.02 (1.25)	6.7	8.91	12.3	
			SoC	8	8 (100.0)	8.59 (0.69)	7.7	8.50	10.0	
		Week 2	Peg	16	15 (93.8)	11.15 (1.38)	7.9	11.10	12.9	
			SoC	8	8 (100.0)	8.67 (1.86)	6.4	8.65	11.9	
		Week 4	Peg	16	15 (93.8)	11.57 (1.54)	7.5	11.80	13.7	
			SoC	8	7 (87.5)	7.59 (0.95)	6.5	7.90	8.6	
		Week 6	Peg	16	14 (87.5)	11.96 (1.32)	8.8	12.00	14.4	
			SoC	8	8 (100.0)	8.15 (1.62)	5.9	8.15	11.4	
		Week 8	Peg	16	15 (93.8)	12.35 (1.67)	7.8	12.20	15.1	
			SoC	8	8 (100.0)	8.08 (2.00)	5.4	8.55	11.4	
		Week 10	Peg	16	15 (93.8)	12.33 (1.62)	7.7	12.60	14.8	
			SoC	8	8 (100.0)	8.08 (1.86)	5.4	8.30	11.4	
		Week 12	Peg	16	15 (93.8)	11.83 (1.92)	6.7	12.40	14.9	
			SoC	8	8 (100.0)	8.01 (1.92)	5.4	8.45	11.4	
		Week 14	Peg	16	15 (93.8)	11.89 (1.71)	6.8	12.20	14.2	
			SoC	8	8 (100.0)	8.03 (1.90)	5.4	8.40	11.4	
		Week 16	Peg	16	15 (93.8)	11.99 (1.94)	6.7	12.50	14.4	
			SoC	8	8 (100.0)	8.03 (1.87)	5.4	8.20	11.4	
		Week 18	Peg	16	15 (93.8)	12.27 (1.33)	9.1	12.70	14.2	
			SoC	8	8 (100.0)	8.21 (1.94)	5.4	8.55	11.4	
		Week 20	Peg	16	14 (87.5)	12.26 (1.43)	8.3	12.65	13.9	
			SoC	8	8 (100.0)	8.15 (2.19)	5.4	8.55	11.4	
		Week 22	Peg	16	15 (93.8)	12.23 (1.35)	8.7	12.30	14.5	
			SoC	8	8 (100.0)	7.95 (2.04)	5.4	8.55	11.4	
		Week 24	Peg	16	15 (93.8)	12.23 (1.44)	8.2	12.50	14.4	
			SoC	8	8 (100.0)	7.85 (2.02)	5.4	8.20	11.4	
		Week 26	Peg	16	13 (81.3)	12.03 (1.40)	9.2	11.90	13.8	
			SoC	8	6 (75.0)	7.73 (2.37)	5.4	7.30	11.4	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.

95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.

Hb = Hemoglobin levels (g/dl).

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.

Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SIHD: Change from baseline in Hb (censored) by sex
ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in Hb (censored)	Week 2	Peg	19	18 (94.7)	1.60 (1.52)	-0.9	1.61	4.8	1.10 (0.46) [0.30, 2.10]
		SoC	10	9 (90.0)	-0.27 (1.90)	-2.6	-0.49	3.8	
	Week 4	Peg	19	18 (94.7)	2.10 (1.87)	-0.7	2.10	6.6	1.25 (0.45) [0.47, 2.25]
		SoC	10	10 (100.0)	-0.34 (1.92)	-2.0	-0.93	3.4	
	Week 6	Peg	19	19 (100.0)	2.52 (1.88)	-1.2	2.62	7.3	1.16 (0.44) [0.39, 2.12]
		SoC	10	10 (100.0)	0.41 (1.53)	-1.3	0.78	2.4	
	Week 8	Peg	19	18 (94.7)	2.37 (2.43)	-4.6	2.73	7.0	1.26 (0.45) [0.48, 2.26]
		SoC	10	10 (100.0)	-0.49 (1.71)	-2.5	-0.92	2.9	
	Week 10	Peg	19	17 (89.5)	2.51 (2.48)	-3.8	2.97	7.3	0.85 (0.44) [0.07, 1.78]
		SoC	10	10 (100.0)	0.49 (1.98)	-2.1	0.60	3.6	
	Week 12	Peg	19	19 (100.0)	2.41 (2.23)	-2.3	2.23	7.5	0.65 (0.42) [-0.11, 1.53]
		SoC	10	10 (100.0)	0.95 (2.07)	-1.4	0.95	5.5	
	Week 14	Peg	19	19 (100.0)	2.27 (2.41)	-3.6	2.59	7.6	0.95 (0.43) [0.18, 1.87]
		SoC	10	10 (100.0)	0.16 (1.57)	-1.4	-0.53	3.4	
	Week 16	Peg	19	19 (100.0)	2.53 (2.87)	-3.8	3.03	8.1	1.06 (0.43) [0.29, 2.01]
		SoC	10	10 (100.0)	-0.12 (1.11)	-1.4	-0.15	1.9	
	Week 18	Peg	19	19 (100.0)	2.57 (2.54)	-4.6	2.92	7.5	0.76 (0.42) [-0.00, 1.66]
		SoC	10	10 (100.0)	0.78 (1.63)	-1.4	0.95	3.5	
	Week 20	Peg	19	19 (100.0)	3.00 (2.13)	-0.3	3.16	8.0	1.19 (0.46) [0.39, 2.19]
		SoC	10	9 (90.0)	0.40 (2.10)	-1.9	0.25	3.7	
	Week 22	Peg	19	18 (94.7)	3.29 (2.30)	-0.0	3.45	8.0	1.10 (0.46) [0.30, 2.10]
		SoC	10	9 (90.0)	0.83 (1.84)	-1.4	0.94	3.8	
	Week 24	Peg	19	19 (100.0)	3.15 (2.72)	-1.9	3.03	8.5	0.94 (0.43) [0.18, 1.86]
		SoC	10	10 (100.0)	0.67 (2.20)	-1.7	0.90	5.6	
	Week 26	Peg	19	17 (89.5)	3.53 (2.25)	-0.6	3.70	7.9	1.09 (0.46) [0.28, 2.11]
		SoC	10	9 (90.0)	0.80 (2.71)	-2.0	0.94	6.9	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
Hb = Hemoglobin levels (g/dl).
Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SIHD: Change from baseline in Hb (censored) by sex
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Female in Hb (censored)	Week 2	Peg	16	15 (93.8)	2.11 (1.08)	0.5	1.95	4.0	1.52 (0.53) [0.64, 2.72]
		SoC	8	8 (100.0)	0.09 (1.62)	-2.3	0.30	1.9	
	Week 4	Peg	16	15 (93.8)	2.53 (1.28)	0.1	2.23	4.6	2.89 (0.70) [1.85, 4.58]
		SoC	8	7 (87.5)	-1.01 (0.89)	-2.1	-1.22	0.3	
	Week 6	Peg	16	14 (87.5)	2.96 (1.12)	1.4	3.08	5.1	2.89 (0.69) [1.86, 4.55]
		SoC	8	8 (100.0)	-0.43 (1.16)	-2.6	-0.46	1.4	
	Week 8	Peg	16	15 (93.8)	3.31 (1.34)	0.4	3.45	5.4	2.39 (0.61) [1.44, 3.84]
		SoC	8	8 (100.0)	-0.51 (1.88)	-3.1	-0.08	1.5	
	Week 10	Peg	16	15 (93.8)	3.28 (1.55)	0.3	3.40	6.5	2.32 (0.61) [1.38, 3.76]
		SoC	8	8 (100.0)	-0.51 (1.63)	-3.1	-0.08	1.4	
	Week 12	Peg	16	15 (93.8)	2.79 (1.70)	-0.8	2.95	6.6	1.90 (0.56) [1.00, 3.20]
		SoC	8	8 (100.0)	-0.57 (1.73)	-3.1	-0.08	1.4	
	Week 14	Peg	16	15 (93.8)	2.85 (1.45)	-0.6	3.10	5.0	2.14 (0.59) [1.22, 3.51]
		SoC	8	8 (100.0)	-0.56 (1.70)	-3.1	-0.08	1.4	
	Week 16	Peg	16	15 (93.8)	2.95 (1.66)	-0.8	3.25	5.9	2.08 (0.58) [1.16, 3.44]
		SoC	8	8 (100.0)	-0.56 (1.57)	-3.1	-0.50	1.4	
	Week 18	Peg	16	15 (93.8)	3.23 (1.37)	0.6	3.10	6.0	2.26 (0.60) [1.33, 3.68]
		SoC	8	8 (100.0)	-0.38 (1.82)	-3.1	-0.08	1.8	
	Week 20	Peg	16	14 (87.5)	3.21 (1.46)	0.9	3.25	6.0	2.03 (0.59) [1.11, 3.40]
		SoC	8	8 (100.0)	-0.44 (2.15)	-3.1	-0.08	2.6	
	Week 22	Peg	16	15 (93.8)	3.18 (1.28)	0.8	3.30	6.0	2.44 (0.62) [1.49, 3.91]
		SoC	8	8 (100.0)	-0.64 (1.88)	-3.1	-0.08	1.4	
	Week 24	Peg	16	15 (93.8)	3.18 (1.36)	0.8	3.35	6.5	2.48 (0.62) [1.52, 3.96]
		SoC	8	8 (100.0)	-0.74 (1.81)	-3.1	-0.15	1.4	
	Week 26	Peg	16	13 (81.3)	2.68 (1.28)	0.3	2.70	4.3	2.25 (0.68) [1.21, 3.88]
		SoC	8	6 (75.0)	-1.00 (2.10)	-3.1	-1.45	1.5	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.

95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.

Hb = Hemoglobin levels (g/dl).

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.

Source Data: aeFF, created on: 13SEP2023

Table PT1HBWC_SICB: Change from baseline in Hb (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.8277

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Hb = Hemoglobin levels (g/dl).
 Source Data: aeфф, created on: 13SEP2023

Table PT1HBWC_SICB: Change from baseline in Hb (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Latin America	Peg	Week 2	12	11 (91.7)	1.83 (0.38)	(0.97, 2.69)	2.26 (0.97)	(0.07, 4.45)	0.0448	*
		Week 4	12	11 (91.7)	2.39 (0.46)	(1.34, 3.44)	4.10 (1.18)	(1.42, 6.78)	0.0071	*
		Week 6	12	10 (83.3)	2.33 (0.60)	(0.95, 3.71)	2.25 (1.44)	(-1.08, 5.58)	0.1581	
		Week 8	12	10 (83.3)	2.92 (0.90)	(0.84, 5.01)	2.58 (2.22)	(-2.53, 7.69)	0.2781	
		Week 10	12	9 (75.0)	2.93 (0.96)	(0.67, 5.19)	2.54 (2.23)	(-2.74, 7.83)	0.2922	
		Week 12	12	11 (91.7)	2.71 (0.76)	(0.99, 4.44)	2.59 (1.95)	(-1.82, 7.00)	0.2170	
		Week 14	12	11 (91.7)	2.75 (0.84)	(0.85, 4.65)	2.58 (2.14)	(-2.26, 7.43)	0.2582	
		Week 16	12	11 (91.7)	2.76 (0.92)	(0.68, 4.85)	2.66 (2.35)	(-2.65, 7.97)	0.2867	
		Week 18	12	11 (91.7)	2.91 (0.94)	(0.78, 5.04)	2.76 (2.40)	(-2.68, 8.19)	0.2812	
		Week 20	12	11 (91.7)	3.54 (0.59)	(2.20, 4.88)	3.41 (1.51)	(-0.00, 6.82)	0.0501	
		Week 22	12	11 (91.7)	3.64 (0.60)	(2.29, 4.99)	3.48 (1.52)	(0.04, 6.92)	0.0481	*
		Week 24	12	11 (91.7)	3.48 (0.72)	(1.85, 5.11)	3.39 (1.84)	(-0.77, 7.54)	0.0986	
		Week 26	12	10 (83.3)	3.61 (0.61)	(2.21, 5.01)	3.50 (1.48)	(0.09, 6.90)	0.0453	*
			SoC	Week 2	2	2 (100.0)	-0.43 (0.89)	(-2.45, 1.58)		
	Week 4	2		2 (100.0)	-1.71 (1.09)	(-4.17, 0.75)				
	Week 6	2		2 (100.0)	0.08 (1.32)	(-2.95, 3.12)				
	Week 8	2		2 (100.0)	0.35 (2.02)	(-4.32, 5.01)				
	Week 10	2		2 (100.0)	0.39 (2.02)	(-4.39, 5.16)				
	Week 12	2		2 (100.0)	0.13 (1.79)	(-3.93, 4.18)				
	Week 14	2		2 (100.0)	0.17 (1.97)	(-4.28, 4.62)				
	Week 16	2		2 (100.0)	0.11 (2.16)	(-4.77, 4.99)				
	Week 18	2		2 (100.0)	0.16 (2.21)	(-4.84, 5.15)				
	Week 20	2		2 (100.0)	0.13 (1.39)	(-3.01, 3.27)				
	Week 22	2	2 (100.0)	0.16 (1.40)	(-3.00, 3.32)					
	Week 24	2	2 (100.0)	0.10 (1.69)	(-3.72, 3.92)					
	Week 26	2	2 (100.0)	0.11 (1.34)	(-2.98, 3.21)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.

ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.

ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to

Peg. NE = not evaluable. IA = p-value of interaction term.

Hb = Hemoglobin levels (g/dl).

Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SICB: Change from baseline in Hb (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Asia Pacific Peg		Week 2	23	22 (95.7)	1.84 (0.32)	(1.20, 2.49)	1.98 (0.52)	(0.93, 3.04)	0.0005	*
		Week 4	23	22 (95.7)	2.26 (0.33)	(1.59, 2.93)	2.88 (0.53)	(1.80, 3.96)	<0.0001	*
		Week 6	23	23 (100.0)	2.76 (0.27)	(2.21, 3.31)	2.77 (0.44)	(1.88, 3.66)	<0.0001	*
		Week 8	23	23 (100.0)	2.74 (0.33)	(2.06, 3.41)	3.42 (0.54)	(2.33, 4.52)	<0.0001	*
		Week 10	23	23 (100.0)	2.80 (0.38)	(2.03, 3.57)	2.87 (0.61)	(1.63, 4.11)	<0.0001	*
		Week 12	23	23 (100.0)	2.38 (0.37)	(1.63, 3.13)	2.11 (0.60)	(0.90, 3.32)	0.0011	*
		Week 14	23	23 (100.0)	2.36 (0.33)	(1.69, 3.02)	2.64 (0.53)	(1.56, 3.71)	<0.0001	*
		Week 16	23	23 (100.0)	2.63 (0.36)	(1.91, 3.35)	3.05 (0.57)	(1.88, 4.21)	<0.0001	*
		Week 18	23	23 (100.0)	2.82 (0.34)	(2.13, 3.52)	2.67 (0.55)	(1.54, 3.79)	<0.0001	*
		Week 20	23	22 (95.7)	2.91 (0.41)	(2.08, 3.75)	3.12 (0.66)	(1.77, 4.47)	<0.0001	*
		Week 22	23	22 (95.7)	3.02 (0.39)	(2.23, 3.81)	2.94 (0.63)	(1.65, 4.22)	<0.0001	*
		Week 24	23	23 (100.0)	2.98 (0.43)	(2.10, 3.86)	3.08 (0.70)	(1.66, 4.50)	<0.0001	*
		Week 26	23	20 (87.0)	2.91 (0.49)	(1.90, 3.91)	2.95 (0.83)	(1.25, 4.64)	0.0013	*
SoC		Week 2	16	15 (93.8)	-0.14 (0.39)	(-0.93, 0.65)				
		Week 4	16	15 (93.8)	-0.62 (0.40)	(-1.43, 0.19)				
		Week 6	16	16 (100.0)	-0.01 (0.33)	(-0.67, 0.65)				
		Week 8	16	16 (100.0)	-0.69 (0.40)	(-1.50, 0.13)				
		Week 10	16	16 (100.0)	-0.07 (0.46)	(-0.99, 0.86)				
		Week 12	16	16 (100.0)	0.27 (0.44)	(-0.63, 1.17)				
		Week 14	16	16 (100.0)	-0.28 (0.39)	(-1.08, 0.52)				
		Week 16	16	16 (100.0)	-0.42 (0.43)	(-1.29, 0.45)				
		Week 18	16	16 (100.0)	0.16 (0.41)	(-0.68, 0.99)				
		Week 20	16	15 (93.8)	-0.21 (0.49)	(-1.21, 0.80)				
		Week 22	16	15 (93.8)	0.08 (0.47)	(-0.88, 1.05)				
		Week 24	16	16 (100.0)	-0.10 (0.52)	(-1.16, 0.96)				
		Week 26	16	13 (81.3)	-0.04 (0.62)	(-1.31, 1.23)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Hb = Hemoglobin levels (g/dl).
 Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SIHB: Change from baseline in Hb (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Hb level (censored)	Latin America	Baseline	Peg	12	12 (100.0)	9.17 (1.50)	6.5	9.07	13.1	
			SoC	2	2 (100.0)	8.87 (0.12)	8.8	8.87	9.0	
		Week 2	Peg	12	11 (91.7)	11.06 (1.47)	8.6	11.30	12.8	
			SoC	2	2 (100.0)	8.64 (0.48)	8.3	8.64	9.0	
		Week 4	Peg	12	11 (91.7)	11.64 (1.75)	8.3	11.90	13.7	
			SoC	2	2 (100.0)	7.40 (0.84)	6.8	7.40	8.0	
		Week 6	Peg	12	10 (83.3)	11.65 (1.91)	7.8	11.89	13.8	
			SoC	2	2 (100.0)	9.18 (2.16)	7.7	9.18	10.7	
		Week 8	Peg	12	10 (83.3)	11.76 (2.84)	4.4	12.62	13.9	
			SoC	2	2 (100.0)	9.18 (2.16)	7.7	9.18	10.7	
		Week 10	Peg	12	9 (75.0)	11.85 (2.84)	5.1	12.80	14.3	
			SoC	2	2 (100.0)	9.18 (2.16)	7.7	9.18	10.7	
		Week 12	Peg	12	11 (91.7)	11.97 (2.58)	6.7	12.80	15.1	
			SoC	2	2 (100.0)	9.18 (2.16)	7.7	9.18	10.7	
		Week 14	Peg	12	11 (91.7)	12.04 (2.95)	5.4	13.10	16.6	
			SoC	2	2 (100.0)	9.18 (2.16)	7.7	9.18	10.7	
		Week 16	Peg	12	11 (91.7)	12.08 (3.35)	5.4	13.00	16.1	
			SoC	2	2 (100.0)	9.18 (2.16)	7.7	9.18	10.7	
		Week 18	Peg	12	11 (91.7)	12.15 (3.06)	4.4	13.20	16.0	
			SoC	2	2 (100.0)	9.18 (2.16)	7.7	9.18	10.7	
		Week 20	Peg	12	11 (91.7)	12.76 (1.89)	9.2	12.80	16.2	
			SoC	2	2 (100.0)	9.18 (2.16)	7.7	9.18	10.7	
		Week 22	Peg	12	11 (91.7)	12.89 (2.10)	9.7	12.90	17.1	
			SoC	2	2 (100.0)	9.18 (2.16)	7.7	9.18	10.7	
		Week 24	Peg	12	11 (91.7)	12.76 (2.55)	7.1	13.10	16.1	
			SoC	2	2 (100.0)	9.18 (2.16)	7.7	9.18	10.7	
		Week 26	Peg	12	10 (83.3)	12.94 (1.92)	9.6	12.95	16.1	
			SoC	2	2 (100.0)	9.18 (2.16)	7.7	9.18	10.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Hb = Hemoglobin levels (g/dl).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeFF, created on: 13SEP2023

Table PT1HBWC_SIHB: Change from baseline in Hb (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Hb level (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	9.51 (1.37)	6.7	9.21	12.3	
			SoC	16	16 (100.0)	8.65 (0.82)	7.2	8.53	10.0	
		Week 2	Peg	23	22 (95.7)	11.25 (1.67)	7.8	11.25	14.4	
			SoC	16	15 (93.8)	8.57 (1.79)	5.9	8.60	11.9	
		Week 4	Peg	23	22 (95.7)	11.76 (1.93)	7.5	11.88	16.0	
			SoC	16	15 (93.8)	8.16 (1.51)	6.5	7.90	10.8	
		Week 6	Peg	23	23 (100.0)	12.32 (1.75)	8.3	12.10	15.6	
			SoC	16	16 (100.0)	8.65 (1.64)	5.9	8.30	11.4	
		Week 8	Peg	23	23 (100.0)	12.25 (1.95)	7.8	12.44	15.6	
			SoC	16	16 (100.0)	8.05 (1.64)	5.4	7.90	11.4	
		Week 10	Peg	23	23 (100.0)	12.30 (2.07)	7.7	12.50	15.7	
			SoC	16	16 (100.0)	8.67 (1.87)	5.4	8.55	11.8	
		Week 12	Peg	23	23 (100.0)	12.00 (2.08)	6.7	12.40	14.9	
			SoC	16	16 (100.0)	8.92 (2.25)	5.4	8.45	14.0	
		Week 14	Peg	23	23 (100.0)	11.90 (2.01)	6.8	12.20	14.7	
			SoC	16	16 (100.0)	8.43 (1.61)	5.4	8.30	11.4	
		Week 16	Peg	23	23 (100.0)	12.15 (2.21)	6.7	12.60	15.6	
			SoC	16	16 (100.0)	8.26 (1.50)	5.4	8.20	11.4	
		Week 18	Peg	23	23 (100.0)	12.33 (1.85)	8.7	12.70	15.4	
			SoC	16	16 (100.0)	8.91 (1.87)	5.4	8.85	11.5	
		Week 20	Peg	23	22 (95.7)	12.40 (2.02)	8.3	12.75	15.6	
			SoC	16	15 (93.8)	8.63 (1.89)	5.4	8.60	11.4	
		Week 22	Peg	23	22 (95.7)	12.45 (2.10)	8.6	12.43	15.8	
			SoC	16	15 (93.8)	8.78 (1.94)	5.4	8.70	11.4	
		Week 24	Peg	23	23 (100.0)	12.49 (2.27)	8.0	12.90	16.5	
			SoC	16	16 (100.0)	8.66 (2.11)	5.4	8.55	13.0	
		Week 26	Peg	23	20 (87.0)	12.67 (2.26)	8.0	12.90	16.2	
			SoC	16	13 (81.3)	8.79 (2.47)	5.4	8.90	14.4	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.

95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.

Hb = Hemoglobin levels (g/dl).

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.

Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SIHB: Change from baseline in Hb (censored) by region
ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline in Hb (censored)	Latin America	Week 2	Peg	12	11 (91.7)	1.84 (1.66)	-0.9	2.09	4.8	1.21 (0.91)	[-0.30, 3.28]
			SoC	2	2 (100.0)	-0.23 (0.37)	-0.5	-0.23	0.0		
		Week 4	Peg	12	11 (91.7)	2.42 (2.00)	-0.7	2.29	6.6	1.88 (0.98)	[0.38, 4.23]
			SoC	2	2 (100.0)	-1.47 (0.73)	-2.0	-1.47	-1.0		
		Week 6	Peg	12	10 (83.3)	2.46 (2.24)	-1.2	2.24	7.3	0.88 (0.90)	[-0.68, 2.89]
			SoC	2	2 (100.0)	0.32 (2.27)	-1.3	0.32	1.9		
		Week 8	Peg	12	10 (83.3)	2.93 (2.99)	-4.6	3.43	7.0	0.82 (0.90)	[-0.75, 2.81]
			SoC	2	2 (100.0)	0.32 (2.27)	-1.3	0.32	1.9		
		Week 10	Peg	12	9 (75.0)	3.06 (3.03)	-3.8	3.40	7.3	0.85 (0.92)	[-0.74, 2.92]
			SoC	2	2 (100.0)	0.32 (2.27)	-1.3	0.32	1.9		
		Week 12	Peg	12	11 (91.7)	2.76 (2.61)	-2.3	3.20	7.5	0.88 (0.88)	[-0.67, 2.83]
			SoC	2	2 (100.0)	0.32 (2.27)	-1.3	0.32	1.9		
		Week 14	Peg	12	11 (91.7)	2.82 (2.83)	-3.6	3.47	7.6	0.84 (0.88)	[-0.71, 2.77]
			SoC	2	2 (100.0)	0.32 (2.27)	-1.3	0.32	1.9		
		Week 16	Peg	12	11 (91.7)	2.86 (3.36)	-3.8	3.45	8.1	0.72 (0.87)	[-0.84, 2.62]
			SoC	2	2 (100.0)	0.32 (2.27)	-1.3	0.32	1.9		
		Week 18	Peg	12	11 (91.7)	2.93 (3.02)	-4.6	3.60	7.5	0.82 (0.88)	[-0.73, 2.75]
			SoC	2	2 (100.0)	0.32 (2.27)	-1.3	0.32	1.9		
		Week 20	Peg	12	11 (91.7)	3.54 (1.98)	-0.0	3.20	8.0	1.49 (0.93)	[-0.01, 3.67]
			SoC	2	2 (100.0)	0.32 (2.27)	-1.3	0.32	1.9		
		Week 22	Peg	12	11 (91.7)	3.67 (2.01)	0.7	3.95	8.0	1.53 (0.94)	[0.03, 3.73]
			SoC	2	2 (100.0)	0.32 (2.27)	-1.3	0.32	1.9		
		Week 24	Peg	12	11 (91.7)	3.54 (2.63)	-1.9	3.50	8.5	1.15 (0.90)	[-0.37, 3.20]
			SoC	2	2 (100.0)	0.32 (2.27)	-1.3	0.32	1.9		
		Week 26	Peg	12	10 (83.3)	3.70 (2.01)	0.4	3.37	7.9	1.53 (0.96)	[0.02, 3.82]
			SoC	2	2 (100.0)	0.32 (2.27)	-1.3	0.32	1.9		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
Hb = Hemoglobin levels (g/dl).
Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SIHB: Change from baseline in Hb (censored) by region
ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline in Hb (censored)	Asia Pacific	Week 2	Peg	23	22 (95.7)	1.82 (1.20)	-0.8	1.78	4.0	1.25 (0.38) [0.59, 2.08]	
			SoC	16	15 (93.8)	-0.09 (1.84)	-2.6	-0.34	3.8		
			Week 4	Peg	23	22 (95.7)	2.24 (1.45)	-0.7	2.18	4.6	1.75 (0.41) [1.06, 2.67]
				SoC	16	15 (93.8)	-0.50 (1.64)	-2.1	-0.89	3.4	
			Week 6	Peg	23	23 (100.0)	2.81 (1.26)	-0.3	2.97	5.1	2.11 (0.42) [1.40, 3.06]
				SoC	16	16 (100.0)	0.00 (1.37)	-2.6	-0.16	2.4	
			Week 8	Peg	23	23 (100.0)	2.74 (1.54)	-0.3	2.93	5.4	2.03 (0.42) [1.33, 2.96]
				SoC	16	16 (100.0)	-0.60 (1.72)	-3.1	-0.52	2.9	
			Week 10	Peg	23	23 (100.0)	2.80 (1.69)	-0.1	3.03	6.5	1.54 (0.38) [0.88, 2.39]
				SoC	16	16 (100.0)	0.01 (1.88)	-3.1	0.29	3.6	
			Week 12	Peg	23	23 (100.0)	2.49 (1.69)	-0.8	2.63	6.6	1.18 (0.36) [0.53, 1.96]
				SoC	16	16 (100.0)	0.27 (2.08)	-3.1	0.50	5.5	
			Week 14	Peg	23	23 (100.0)	2.39 (1.59)	-0.6	2.43	5.0	1.60 (0.39) [0.93, 2.46]
				SoC	16	16 (100.0)	-0.22 (1.61)	-3.1	-0.47	3.4	
			Week 16	Peg	23	23 (100.0)	2.64 (1.84)	-0.8	2.79	6.2	1.83 (0.40) [1.15, 2.73]
				SoC	16	16 (100.0)	-0.39 (1.24)	-3.1	-0.46	1.4	
			Week 18	Peg	23	23 (100.0)	2.82 (1.57)	-0.3	2.99	6.0	1.51 (0.38) [0.85, 2.35]
				SoC	16	16 (100.0)	0.26 (1.79)	-3.1	0.64	3.5	
			Week 20	Peg	23	22 (95.7)	2.87 (1.79)	-0.3	3.23	6.2	1.46 (0.39) [0.78, 2.32]
				SoC	16	15 (93.8)	-0.03 (2.16)	-3.1	0.25	3.7	
			Week 22	Peg	23	22 (95.7)	3.03 (1.82)	-0.0	2.84	6.3	1.50 (0.39) [0.83, 2.37]
				SoC	16	15 (93.8)	0.12 (2.00)	-3.1	0.65	3.8	
			Week 24	Peg	23	23 (100.0)	2.98 (2.00)	-1.1	2.99	7.1	1.41 (0.38) [0.75, 2.23]
				SoC	16	16 (100.0)	0.01 (2.16)	-3.1	0.25	5.6	
			Week 26	Peg	23	20 (87.0)	2.89 (1.86)	-0.6	2.61	6.8	1.25 (0.41) [0.54, 2.14]
				SoC	16	13 (81.3)	0.05 (2.70)	-3.1	-0.49	6.9	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
Hb = Hemoglobin levels (g/dl).
Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SICA: Change from baseline in Hb (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.7400

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Hb = Hemoglobin levels (g/dl).
 Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SICA: Change from baseline in Hb (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
< 4	Peg	Week 2	21	19 (90.5)	2.28 (0.30)	(1.66, 2.90)	2.38 (0.60)	(1.13, 3.62)	0.0007	*
		Week 4	21	19 (90.5)	2.81 (0.31)	(2.16, 3.46)	3.40 (0.61)	(2.15, 4.66)	<0.0001	*
		Week 6	21	20 (95.2)	3.41 (0.26)	(2.87, 3.95)	3.65 (0.51)	(2.59, 4.71)	<0.0001	*
		Week 8	21	19 (90.5)	3.70 (0.25)	(3.18, 4.22)	4.46 (0.48)	(3.46, 5.46)	<0.0001	*
		Week 10	21	19 (90.5)	3.78 (0.33)	(3.10, 4.46)	4.35 (0.63)	(3.05, 5.66)	<0.0001	*
		Week 12	21	20 (95.2)	3.43 (0.38)	(2.65, 4.21)	2.92 (0.74)	(1.39, 4.45)	0.0006	*
		Week 14	21	20 (95.2)	3.44 (0.29)	(2.85, 4.03)	3.93 (0.56)	(2.77, 5.09)	<0.0001	*
		Week 16	21	20 (95.2)	3.56 (0.29)	(2.96, 4.16)	4.33 (0.57)	(3.15, 5.51)	<0.0001	*
		Week 18	21	20 (95.2)	3.43 (0.34)	(2.73, 4.13)	3.14 (0.67)	(1.76, 4.51)	<0.0001	*
		Week 20	21	20 (95.2)	3.63 (0.31)	(2.99, 4.26)	3.93 (0.63)	(2.62, 5.23)	<0.0001	*
		Week 22	21	19 (90.5)	3.93 (0.37)	(3.16, 4.69)	3.53 (0.74)	(1.99, 5.07)	<0.0001	*
		Week 24	21	20 (95.2)	3.90 (0.43)	(3.01, 4.79)	3.77 (0.85)	(2.02, 5.51)	0.0002	*
		Week 26	21	18 (85.7)	3.94 (0.45)	(3.01, 4.86)	3.67 (0.95)	(1.70, 5.65)	0.0009	*
		SoC	Week 2	8	7 (87.5)	-0.10 (0.51)	(-1.15, 0.95)			
	Week 4		8	8 (100.0)	-0.59 (0.50)	(-1.62, 0.44)				
	Week 6		8	8 (100.0)	-0.24 (0.43)	(-1.12, 0.64)				
	Week 8		8	8 (100.0)	-0.75 (0.40)	(-1.58, 0.07)				
	Week 10		8	8 (100.0)	-0.57 (0.52)	(-1.65, 0.50)				
	Week 12		8	8 (100.0)	0.51 (0.62)	(-0.77, 1.78)				
	Week 14		8	8 (100.0)	-0.50 (0.47)	(-1.46, 0.46)				
	Week 16		8	8 (100.0)	-0.77 (0.48)	(-1.75, 0.21)				
	Week 18		8	8 (100.0)	0.30 (0.55)	(-0.84, 1.43)				
	Week 20		8	7 (87.5)	-0.30 (0.53)	(-1.40, 0.80)				
	Week 22		8	7 (87.5)	0.39 (0.63)	(-0.90, 1.69)				
	Week 24		8	8 (100.0)	0.13 (0.70)	(-1.31, 1.58)				
	Week 26	8	6 (75.0)	0.26 (0.81)	(-1.41, 1.94)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.

ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.

ANCOVA: fixed effects = treatment, covariate = baseline.

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to

Peg. NE = not evaluable. IA = p-value of interaction term.

Hb = Hemoglobin levels (g/dl).

Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SICA: Change from baseline in Hb (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 2	14	14 (100.0)	1.46 (0.38)	(0.67, 2.25)	1.89 (0.59)	(0.67, 3.12)	0.0042	*
		Week 4	14	14 (100.0)	1.91 (0.41)	(1.04, 2.77)	3.03 (0.66)	(1.65, 4.41)	0.0002	*
		Week 6	14	13 (92.9)	1.93 (0.42)	(1.06, 2.81)	2.08 (0.64)	(0.75, 3.41)	0.0039	*
		Week 8	14	14 (100.0)	1.83 (0.63)	(0.52, 3.14)	2.48 (0.98)	(0.45, 4.51)	0.0190	*
		Week 10	14	13 (92.9)	1.87 (0.62)	(0.58, 3.15)	1.74 (0.93)	(-0.20, 3.69)	0.0767	
		Week 12	14	14 (100.0)	1.62 (0.54)	(0.49, 2.74)	1.88 (0.84)	(0.14, 3.62)	0.0353	*
		Week 14	14	14 (100.0)	1.51 (0.58)	(0.30, 2.71)	1.78 (0.90)	(-0.09, 3.66)	0.0610	
		Week 16	14	14 (100.0)	1.77 (0.69)	(0.34, 3.19)	2.08 (1.06)	(-0.13, 4.29)	0.0638	
		Week 18	14	14 (100.0)	2.30 (0.64)	(0.98, 3.62)	2.42 (0.99)	(0.37, 4.46)	0.0231	*
		Week 20	14	13 (92.9)	2.64 (0.62)	(1.35, 3.93)	2.89 (0.94)	(0.93, 4.85)	0.0060	*
		Week 22	14	14 (100.0)	2.56 (0.52)	(1.47, 3.64)	2.93 (0.81)	(1.25, 4.61)	0.0016	*
		Week 24	14	14 (100.0)	2.44 (0.58)	(1.23, 3.65)	2.93 (0.90)	(1.05, 4.81)	0.0039	*
		Week 26	14	12 (85.7)	2.37 (0.60)	(1.11, 3.63)	2.91 (0.92)	(0.97, 4.84)	0.0055	*
		SoC	Week 2	10	10 (100.0)	-0.43 (0.45)	(-1.37, 0.50)			
	Week 4		10	9 (90.0)	-1.12 (0.52)	(-2.20, -0.05)				
	Week 6		10	10 (100.0)	-0.15 (0.48)	(-1.15, 0.85)				
	Week 8		10	10 (100.0)	-0.65 (0.75)	(-2.20, 0.90)				
	Week 10		10	10 (100.0)	0.12 (0.70)	(-1.34, 1.59)				
	Week 12		10	10 (100.0)	-0.27 (0.64)	(-1.60, 1.06)				
	Week 14		10	10 (100.0)	-0.28 (0.69)	(-1.71, 1.15)				
	Week 16		10	10 (100.0)	-0.31 (0.81)	(-2.00, 1.37)				
	Week 18		10	10 (100.0)	-0.12 (0.75)	(-1.68, 1.45)				
	Week 20		10	10 (100.0)	-0.25 (0.71)	(-1.73, 1.22)				
	Week 22		10	10 (100.0)	-0.37 (0.62)	(-1.65, 0.91)				
	Week 24		10	10 (100.0)	-0.48 (0.69)	(-1.92, 0.95)				
	Week 26	10	9 (90.0)	-0.53 (0.70)	(-1.99, 0.93)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.

ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.

ANCOVA: fixed effects = treatment, covariate = baseline.

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to

Peg. NE = not evaluable. IA = p-value of interaction term.

Hb = Hemoglobin levels (g/dl).

Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SIHA: Change from baseline in Hb (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Hb level (censored)	< 4	Baseline	Peg	21	21 (100.0)	9.72 (1.67)	6.5	9.56	13.1	
			SoC	8	8 (100.0)	8.50 (0.87)	7.2	8.50	9.8	
	Week 2	Peg	21	19 (90.5)	11.79 (1.27)	9.9	11.60	14.4		
		SoC	8	7 (87.5)	8.89 (1.83)	5.9	8.98	11.0		
	Week 4	Peg	21	19 (90.5)	12.38 (1.42)	10.6	12.10	16.0		
		SoC	8	8 (100.0)	8.47 (1.49)	6.6	8.20	10.7		
	Week 6	Peg	21	20 (95.2)	12.97 (1.25)	11.0	12.98	15.6		
		SoC	8	8 (100.0)	8.78 (1.43)	7.3	8.30	11.2		
	Week 8	Peg	21	19 (90.5)	13.11 (1.18)	11.3	12.94	15.6		
		SoC	8	8 (100.0)	8.19 (1.29)	6.4	7.90	10.1		
	Week 10	Peg	21	19 (90.5)	13.14 (1.48)	9.9	13.20	15.7		
		SoC	8	8 (100.0)	8.48 (1.32)	6.4	8.50	11.0		
	Week 12	Peg	21	20 (95.2)	13.01 (1.51)	9.7	13.05	15.1		
		SoC	8	8 (100.0)	9.48 (2.37)	7.3	8.40	14.0		
	Week 14	Peg	21	20 (95.2)	13.01 (1.51)	10.7	12.95	16.6		
		SoC	8	8 (100.0)	8.50 (1.07)	7.7	8.05	10.8		
	Week 16	Peg	21	20 (95.2)	13.14 (1.64)	9.8	13.45	16.1		
		SoC	8	8 (100.0)	8.21 (0.71)	7.0	8.25	9.1		
	Week 18	Peg	21	20 (95.2)	13.02 (1.63)	9.8	13.09	16.0		
		SoC	8	8 (100.0)	9.24 (1.67)	7.1	9.05	11.5		
	Week 20	Peg	21	20 (95.2)	13.16 (1.42)	11.0	12.85	16.2		
		SoC	8	7 (87.5)	8.87 (1.30)	7.7	8.60	10.9		
	Week 22	Peg	21	19 (90.5)	13.44 (1.76)	10.1	13.40	17.1		
		SoC	8	7 (87.5)	9.38 (1.47)	7.7	9.10	11.4		
	Week 24	Peg	21	20 (95.2)	13.43 (1.91)	8.0	13.35	16.1		
		SoC	8	8 (100.0)	9.23 (1.94)	7.4	8.65	13.0		
	Week 26	Peg	21	18 (85.7)	13.65 (1.63)	10.7	13.65	16.1		
		SoC	8	6 (75.0)	9.60 (2.46)	7.7	8.95	14.4		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.

95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.

Hb = Hemoglobin levels (g/dl).

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.

Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SIHA: Change from baseline in Hb (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Hb level (censored)	>= 4	Baseline	Peg	14	14 (100.0)	8.90 (0.63)	7.4	8.94	9.9	
			SoC	10	10 (100.0)	8.82 (0.70)	7.7	8.73	10.0	
	Week 2	Peg	14	14 (100.0)	10.37 (1.64)	7.8	10.45	12.7		
		SoC	10	10 (100.0)	8.37 (1.63)	6.4	8.15	11.9		
	Week 4	Peg	14	14 (100.0)	10.82 (2.01)	7.5	11.50	13.7		
		SoC	10	9 (90.0)	7.71 (1.39)	6.5	7.30	10.8		
	Week 6	Peg	14	13 (92.9)	10.80 (1.75)	7.8	11.00	13.2		
		SoC	10	10 (100.0)	8.66 (1.86)	5.9	8.30	11.4		
	Week 8	Peg	14	14 (100.0)	10.73 (2.59)	4.4	11.10	13.9		
		SoC	10	10 (100.0)	8.17 (1.99)	5.4	8.15	11.4		
	Week 10	Peg	14	13 (92.9)	10.76 (2.53)	5.1	11.00	13.4		
		SoC	10	10 (100.0)	8.92 (2.22)	5.4	8.80	11.8		
	Week 12	Peg	14	14 (100.0)	10.54 (2.29)	6.7	10.45	14.0		
		SoC	10	10 (100.0)	8.53 (2.03)	5.4	8.45	11.4		
	Week 14	Peg	14	14 (100.0)	10.42 (2.45)	5.4	10.82	13.1		
		SoC	10	10 (100.0)	8.53 (2.02)	5.4	8.40	11.4		
	Week 16	Peg	14	14 (100.0)	10.68 (3.02)	5.4	11.55	15.6		
		SoC	10	10 (100.0)	8.49 (2.00)	5.4	8.20	11.4		
	Week 18	Peg	14	14 (100.0)	11.20 (2.64)	4.4	11.78	14.8		
		SoC	10	10 (100.0)	8.70 (2.00)	5.4	8.85	11.4		
	Week 20	Peg	14	13 (92.9)	11.54 (2.31)	8.3	12.00	15.6		
		SoC	10	10 (100.0)	8.57 (2.23)	5.4	8.80	11.4		
	Week 22	Peg	14	14 (100.0)	11.46 (1.98)	8.6	11.50	15.7		
		SoC	10	10 (100.0)	8.44 (2.14)	5.4	8.60	11.4		
	Week 24	Peg	14	14 (100.0)	11.36 (2.40)	7.1	11.30	16.5		
		SoC	10	10 (100.0)	8.32 (2.15)	5.4	8.20	11.4		
	Week 26	Peg	14	12 (85.7)	11.41 (2.12)	8.0	11.35	16.2		
		SoC	10	9 (90.0)	8.34 (2.30)	5.4	8.50	11.4		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Hb = Hemoglobin levels (g/dl).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SIHA: Change from baseline in Hb (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in Hb (censored)	Week 2	Peg	21	19 (90.5)	2.10 (1.33)	-0.9	2.22	4.8	1.08 (0.49) [0.21, 2.15]
		SoC	8	7 (87.5)	0.39 (2.02)	-2.6	0.03	3.8	
	Week 4	Peg	21	19 (90.5)	2.58 (1.49)	0.1	2.20	6.6	1.56 (0.50) [0.72, 2.69]
		SoC	8	8 (100.0)	-0.03 (1.90)	-2.0	-0.62	3.4	
	Week 6	Peg	21	20 (95.2)	3.20 (1.41)	0.5	3.13	7.3	1.99 (0.53) [1.13, 3.20]
		SoC	8	8 (100.0)	0.28 (1.47)	-1.3	0.24	2.4	
	Week 8	Peg	21	19 (90.5)	3.52 (1.24)	1.4	3.39	7.0	2.81 (0.61) [1.85, 4.26]
		SoC	8	8 (100.0)	-0.31 (1.51)	-2.1	-0.50	2.9	
	Week 10	Peg	21	19 (90.5)	3.55 (1.59)	0.7	3.40	7.3	2.04 (0.54) [1.16, 3.28]
		SoC	8	8 (100.0)	-0.02 (1.95)	-2.1	-0.52	3.6	
	Week 12	Peg	21	20 (95.2)	3.24 (1.63)	0.8	3.08	7.5	1.22 (0.47) [0.39, 2.25]
		SoC	8	8 (100.0)	0.97 (2.22)	-1.4	0.64	5.5	
	Week 14	Peg	21	20 (95.2)	3.24 (1.46)	1.0	3.30	7.6	2.12 (0.54) [1.24, 3.35]
		SoC	8	8 (100.0)	-0.00 (1.55)	-1.4	-0.53	3.4	
	Week 16	Peg	21	20 (95.2)	3.37 (1.67)	1.2	3.12	8.1	2.39 (0.56) [1.49, 3.70]
		SoC	8	8 (100.0)	-0.29 (0.78)	-1.4	-0.15	0.7	
	Week 18	Peg	21	20 (95.2)	3.25 (1.61)	0.6	3.10	7.5	1.49 (0.49) [0.66, 2.58]
		SoC	8	8 (100.0)	0.74 (1.72)	-1.4	0.64	3.5	
	Week 20	Peg	21	20 (95.2)	3.39 (1.56)	0.9	3.18	8.0	1.69 (0.53) [0.81, 2.87]
		SoC	8	7 (87.5)	0.38 (2.19)	-1.9	0.25	3.7	
	Week 22	Peg	21	19 (90.5)	3.74 (1.74)	0.8	3.85	8.0	1.55 (0.52) [0.67, 2.72]
		SoC	8	7 (87.5)	0.89 (1.91)	-1.4	0.65	3.8	
	Week 24	Peg	21	20 (95.2)	3.66 (2.03)	-1.1	3.40	8.5	1.35 (0.48) [0.53, 2.42]
		SoC	8	8 (100.0)	0.73 (2.28)	-1.4	0.59	5.6	
	Week 26	Peg	21	18 (85.7)	3.67 (1.69)	0.7	3.55	7.9	1.20 (0.53) [0.27, 2.37]
		SoC	8	6 (75.0)	1.05 (3.15)	-1.4	0.22	6.9	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.

95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.

Hb = Hemoglobin levels (g/dl).

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.

Source Data: aeff, created on: 13SEP2023

Table PT1HBWC_SIHA: Change from baseline in Hb (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in Hb (censored)	Week 2	Peg	14	14 (100.0)	1.47 (1.33)	-0.8	1.57	3.5	1.32 (0.49) [0.50, 2.42]
		SoC	10	10 (100.0)	-0.45 (1.50)	-2.4	-0.50	1.9	
	Week 4	Peg	14	14 (100.0)	1.91 (1.77)	-0.7	2.18	4.1	1.91 (0.55) [1.03, 3.20]
		SoC	10	9 (90.0)	-1.14 (1.07)	-2.1	-1.23	1.1	
	Week 6	Peg	14	13 (92.9)	1.94 (1.61)	-1.2	1.95	4.2	1.33 (0.50) [0.50, 2.45]
		SoC	10	10 (100.0)	-0.16 (1.40)	-2.6	-0.46	1.9	
	Week 8	Peg	14	14 (100.0)	1.82 (2.51)	-4.6	2.12	5.0	1.03 (0.47) [0.22, 2.06]
		SoC	10	10 (100.0)	-0.64 (1.96)	-3.1	-0.97	1.9	
	Week 10	Peg	14	13 (92.9)	1.88 (2.41)	-3.8	2.09	5.1	0.78 (0.46) [-0.04, 1.78]
		SoC	10	10 (100.0)	0.10 (1.88)	-3.1	0.66	2.6	
	Week 12	Peg	14	14 (100.0)	1.63 (2.14)	-2.3	1.84	4.6	0.93 (0.46) [0.12, 1.94]
		SoC	10	10 (100.0)	-0.29 (1.76)	-3.1	0.10	1.9	
	Week 14	Peg	14	14 (100.0)	1.52 (2.36)	-3.6	2.06	4.4	0.82 (0.45) [0.01, 1.80]
		SoC	10	10 (100.0)	-0.29 (1.74)	-3.1	0.05	1.9	
	Week 16	Peg	14	14 (100.0)	1.78 (2.96)	-3.8	2.99	6.2	0.81 (0.45) [-0.00, 1.79]
		SoC	10	10 (100.0)	-0.33 (1.67)	-3.1	-0.50	1.9	
	Week 18	Peg	14	14 (100.0)	2.30 (2.62)	-4.6	2.74	5.4	1.00 (0.46) [0.19, 2.02]
		SoC	10	10 (100.0)	-0.11 (1.80)	-3.1	0.23	1.9	
	Week 20	Peg	14	13 (92.9)	2.64 (2.23)	-0.3	3.49	6.2	1.28 (0.49) [0.45, 2.39]
		SoC	10	10 (100.0)	-0.25 (2.11)	-3.1	0.23	2.6	
	Week 22	Peg	14	14 (100.0)	2.56 (1.90)	-0.0	2.67	6.3	1.49 (0.50) [0.67, 2.63]
		SoC	10	10 (100.0)	-0.38 (1.91)	-3.1	0.23	1.9	
	Week 24	Peg	14	14 (100.0)	2.45 (2.30)	-1.9	2.47	7.1	1.33 (0.49) [0.51, 2.43]
		SoC	10	10 (100.0)	-0.50 (1.88)	-3.1	-0.15	1.9	
	Week 26	Peg	14	12 (85.7)	2.39 (2.04)	-0.6	2.42	6.8	1.39 (0.53) [0.52, 2.60]
		SoC	10	9 (90.0)	-0.56 (2.05)	-3.1	-0.49	1.9	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Hb = Hemoglobin levels (g/dl).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1HBI_SIPC: Hb response at Week 26 by age
 ITT

Variable	Subgroup	Time	Peg		SoC		RR	OR	RD	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb response at Week 26	Age	Week 26									NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 HB response: ≥ 1 g/dL increase in Hb levels from baseline at Week 26.
 IA = p-value of interaction term.
 Source Data: aeffer, created on: 11SEP2023

Table PT1HBI_SIPC: Hb response at Week 26 by age
 ITT

Variable	Subgroup	Time	Peg		SoC		RR [95 % CI]	OR [95 % CI]	RD [95 % CI]	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb response at Week 26	< 60	Week 26	29	20 (69.0) [49.2, 84.7]	13	1 (7.7) [0.2, 36.0]	8.97 [1.86, 273.98]	NE [NE, NE]	61.3 [25.1, 80.7]	0.0005	*

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 HB response: >= 1 g/dL increase in Hb levels from baseline at Week 26.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1HBI_SIPC: Hb response at Week 26 by age
 ITT

Variable	Subgroup	Time	N	Peg	SoC	RR	OR	RD	p-value	IA	
				n (%) [95 % CI]	N						n (%) [95 % CI]
Hb response at Week 26	>= 60	Week 26	6	5 (83.3) [35.9, 99.6]	5	0 (0.0) [NE, NE]	NE [NE, NE]	NE [NE, NE]	83.3 [23.7, 99.6]	0.0152	*

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 HB response: >= 1 g/dL increase in Hb levels from baseline at Week 26.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1HBI_SIPD: Hb response at Week 26 by sex
 ITT

Variable	Subgroup	Time	Peg		SoC		RR	OR	RD	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb response at Week 26	Sex	Week 26									NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 HB response: >= 1 g/dL increase in Hb levels from baseline at Week 26.
 IA = p-value of interaction term.
 Source Data: aeffer, created on: 11SEP2023

Table PT1HBI_SIPD: Hb response at Week 26 by sex
 ITT

Variable	Subgroup	Time	Peg		SoC		RR [95 % CI]	OR [95 % CI]	RD [95 % CI]	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb response at Week 26	Male	Week 26	19	14 (73.7) [48.8, 90.9]	10	1 (10.0) [0.3, 44.5]	7.37 [1.53, 221.65]	NE [NE, NE]	63.7 [18.5, 85.4]	0.0017	*

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 HB response: >= 1 g/dL increase in Hb levels from baseline at Week 26.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1HBI_SIPD: Hb response at Week 26 by sex
 ITT

Variable	Subgroup	Time	Peg		SoC		RR	OR	RD	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb response at Week 26	Female	Week 26	16	11 (68.8) [41.3, 89.0]	8	0 (0.0) [NE, NE]	NE [NE, NE]	NE [NE, NE]	68.8 [23.7, 89.0]	0.0020	*

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 HB response: >= 1 g/dL increase in Hb levels from baseline at Week 26.
 IA = p-value of interaction term.
 Source Data: aeffer, created on: 11SEP2023

Table PT1HBI_SIPB: Hb response at Week 26 by region
 ITT

Variable	Subgroup	Time	Peg		SoC		RR	OR	RD	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb response at Week 26	Region	Week 26									NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 HB response: ≥ 1 g/dL increase in Hb levels from baseline at Week 26.
 IA = p-value of interaction term.
 Source Data: aeffer, created on: 11SEP2023

Table PT1HBI_SIPB: Hb response at Week 26 by region
 ITT

Variable	Subgroup	Time	N	Peg	SoC	RR	OR	RD	p-value	IA
				n (%) [95 % CI]	N		n (%) [95 % CI]	[95 % CI]		
Hb response at Week 26	Latin America	Week 26	12	9 (75.0) [42.8, 94.5]	2	0 (0.0) [NE, NE]	NE [NE, NE]	75.0 [-9.6, 94.5]	0.1099	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 HB response: >= 1 g/dL increase in Hb levels from baseline at Week 26.
 IA = p-value of interaction term.
 Source Data: aeffer, created on: 11SEP2023

Table PT1HBI_SIPB: Hb response at Week 26 by region
 ITT

Variable	Subgroup	Time	N	Peg	SoC	RR [95 % CI]	OR [95 % CI]	RD [95 % CI]	p-value	IA	
				n (%) [95 % CI]	N						n (%) [95 % CI]
Hb response at Week 26	Asia Pacific	Week 26	23	16 (69.6) [47.1, 86.8]	16	1 (6.3) [0.2, 30.2]	11.13 [2.19, 334.32]	NE [NE, NE]	63.3 [29.9, 82.5]	<0.0001	*

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 HB response: >= 1 g/dL increase in Hb levels from baseline at Week 26.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1HBI_SIPA: Hb response at Week 26 by number of transfusions previous year
 ITT

Variable	Subgroup	Time	Peg		SoC		RR	OR	RD	p-value IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]				
Hb response at Week 26	Number of transfusions in the previous year	Week 26								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 HB response: ≥ 1 g/dL increase in Hb levels from baseline at Week 26.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1HBI_SIPA: Hb response at Week 26 by number of transfusions previous year
 ITT

Variable	Subgroup	Time	Peg		SoC		RR [95 % CI]	OR [95 % CI]	RD [95 % CI]	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb response at Week 26	< 4	Week 26	21	17 (81.0) [58.1, 94.6]	8	1 (12.5) [0.3, 52.7]	6.48 [1.54, 202.76]	NE [NE, NE]	68.5 [15.7, 89.2]	0.0014	*

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 HB response: >= 1 g/dL increase in Hb levels from baseline at Week 26.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1HBI_SIPA: Hb response at Week 26 by number of transfusions previous year
 ITT

Variable	Subgroup	Time	Peg		SoC		RR	OR	RD	p-value	IA
			N	n (%) [95 % CI]	N	n (%) [95 % CI]					
Hb response at Week 26	>= 4	Week 26	14	8 (57.1) [28.9, 82.3]	10	0 (0.0) [NE, NE]	NE [NE, NE]	NE [NE, NE]	57.1 [22.1, 82.3]	0.0064	*

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n = number of patients with response.
 RR = relative risk. OR = odds ratio. RD = risk difference. NE = not evaluable.
 95% CI = 95% confidence interval (RR/OR/RD: exact method).
 p-value = Fisher's exact test. * = significant treatment effect. Hb = Hemoglobin levels (g/dl).
 HB response: >= 1 g/dL increase in Hb levels from baseline at Week 26.
 IA = p-value of interaction term.
 Source Data: aeffr, created on: 11SEP2023

Table PT1LDHWC_SICC: Change from baseline in LDH (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.9936

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 LDH = Lactate dehydrogenase levels (U/l).
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SICC: Change from baseline in LDH (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 60	Peg	Week 2	29	28 (96.6)	-1791.46 (93.62)	(-1981.33, -1601.59)	-1745.40 (172.16)	(-2094.56, -1396.25)	<0.0001	*
		Week 4	29	27 (93.1)	-1893.87 (104.62)	(-2106.25, -1681.48)	-1923.64 (190.01)	(-2309.39, -1537.89)	<0.0001	*
		Week 6	29	27 (93.1)	-1808.85 (129.06)	(-2070.61, -1547.10)	-1836.72 (225.89)	(-2294.84, -1378.61)	<0.0001	*
		Week 8	29	28 (96.6)	-1826.12 (130.95)	(-2091.46, -1560.79)	-1914.99 (232.42)	(-2385.92, -1444.06)	<0.0001	*
		Week 10	29	28 (96.6)	-1825.00 (117.79)	(-2063.67, -1586.33)	-1860.63 (209.06)	(-2284.23, -1437.04)	<0.0001	*
		Week 12	29	28 (96.6)	-1817.30 (113.90)	(-2048.09, -1586.52)	-1797.49 (202.16)	(-2207.10, -1387.88)	<0.0001	*
		Week 14	29	28 (96.6)	-1809.14 (121.26)	(-2054.84, -1563.44)	-1863.50 (215.22)	(-2299.59, -1427.42)	<0.0001	*
		Week 16	29	28 (96.6)	-1805.84 (124.27)	(-2057.64, -1554.04)	-1817.04 (220.57)	(-2263.95, -1370.13)	<0.0001	*
		Week 18	29	27 (93.1)	-1764.41 (132.30)	(-2032.73, -1496.10)	-1856.00 (231.46)	(-2325.43, -1386.57)	<0.0001	*
		Week 20	29	28 (96.6)	-1801.37 (188.89)	(-2184.45, -1418.29)	-2015.33 (347.34)	(-2719.78, -1310.89)	<0.0001	*
		Week 22	29	28 (96.6)	-1796.54 (189.36)	(-2180.58, -1412.51)	-2071.58 (348.21)	(-2777.77, -1365.38)	<0.0001	*
		Week 24	29	28 (96.6)	-1777.16 (188.85)	(-2159.81, -1394.51)	-1924.74 (335.18)	(-2603.89, -1245.59)	<0.0001	*
		Week 26	29	24 (82.8)	-1883.16 (207.90)	(-2307.17, -1459.15)	-2229.31 (375.46)	(-2995.06, -1463.57)	<0.0001	*
		SoC	Week 2	13	12 (92.3)	-46.06 (142.94)	(-335.95, 243.84)			
	Week 4		13	12 (92.3)	29.77 (157.24)	(-289.43, 348.98)				
	Week 6		13	13 (100.0)	27.87 (184.22)	(-345.74, 401.48)				
	Week 8		13	13 (100.0)	88.87 (191.17)	(-298.48, 476.22)				
	Week 10		13	13 (100.0)	35.64 (171.95)	(-312.78, 384.05)				
	Week 12		13	13 (100.0)	-19.81 (166.28)	(-356.72, 317.10)				
	Week 14		13	13 (100.0)	54.36 (177.02)	(-304.32, 413.05)				
	Week 16		13	13 (100.0)	11.20 (181.42)	(-356.39, 378.79)				
	Week 18		13	13 (100.0)	91.59 (188.81)	(-291.33, 474.50)				
	Week 20		13	12 (92.3)	213.97 (288.39)	(-370.92, 798.85)				
	Week 22		13	12 (92.3)	275.03 (289.11)	(-311.31, 861.37)				
	Week 24		13	13 (100.0)	147.58 (275.69)	(-411.03, 706.19)				
	Week 26	13	11 (84.6)	346.15 (307.88)	(-281.77, 974.08)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 LDH = Lactate dehydrogenase levels (U/l).
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SICC: Change from baseline in LDH (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 2	6	4 (66.7)	-1985.11 (237.04)	(-2594.44, -1375.78)	-1269.95 (341.43)	(-2147.62, -392.28)	0.0137	*
		Week 4	6	6 (100.0)	-2161.04 (203.07)	(-2641.23, -1680.85)	-1480.94 (326.10)	(-2252.04, -709.83)	0.0027	*
		Week 6	6	6 (100.0)	-2130.98 (129.54)	(-2437.28, -1824.67)	-1347.31 (208.01)	(-1839.19, -855.43)	0.0003	*
		Week 8	6	5 (83.3)	-2142.29 (177.49)	(-2576.58, -1708.00)	-1483.98 (273.12)	(-2152.27, -815.69)	0.0016	*
		Week 10	6	6 (100.0)	-2126.21 (151.42)	(-2484.26, -1768.16)	-1406.30 (243.16)	(-1981.27, -831.33)	0.0007	*
		Week 12	6	6 (100.0)	-2121.93 (152.30)	(-2482.06, -1761.81)	-1466.21 (244.56)	(-2044.51, -887.91)	0.0005	*
		Week 14	6	6 (100.0)	-2110.78 (159.69)	(-2488.38, -1733.18)	-1317.72 (256.43)	(-1924.08, -711.36)	0.0013	*
		Week 16	6	6 (100.0)	-2108.52 (162.45)	(-2492.65, -1724.39)	-1300.23 (260.87)	(-1917.08, -683.38)	0.0016	*
		Week 18	6	6 (100.0)	-2132.24 (155.12)	(-2499.04, -1765.45)	-1463.63 (249.10)	(-2052.65, -874.61)	0.0006	*
		Week 20	6	6 (100.0)	-2090.69 (178.36)	(-2512.45, -1668.92)	-1218.06 (286.42)	(-1895.34, -540.77)	0.0038	*
		Week 22	6	5 (83.3)	-2079.94 (220.89)	(-2620.44, -1539.43)	-1181.41 (339.91)	(-2013.13, -349.68)	0.0132	*
		Week 24	6	6 (100.0)	-2129.30 (152.31)	(-2489.46, -1769.15)	-1457.76 (244.58)	(-2036.11, -879.42)	0.0006	*
		Week 26	6	6 (100.0)	-2247.52 (148.91)	(-2630.30, -1864.74)	-1522.17 (281.33)	(-2245.35, -798.98)	0.0029	*
		SoC	Week 2	5	5 (100.0)	-715.16 (205.65)	(-1243.80, -186.52)			
	Week 4		5	5 (100.0)	-680.10 (222.93)	(-1207.26, -152.95)				
	Week 6		5	5 (100.0)	-783.67 (142.21)	(-1119.93, -447.40)				
	Week 8		5	5 (100.0)	-658.31 (177.49)	(-1092.60, -224.02)				
	Week 10		5	5 (100.0)	-719.91 (166.23)	(-1112.98, -326.84)				
	Week 12		5	5 (100.0)	-655.72 (167.19)	(-1051.07, -260.38)				
	Week 14		5	5 (100.0)	-793.06 (175.31)	(-1207.59, -378.53)				
	Week 16		5	5 (100.0)	-808.29 (178.34)	(-1229.99, -386.59)				
	Week 18		5	5 (100.0)	-668.62 (170.29)	(-1071.29, -265.94)				
	Week 20		5	5 (100.0)	-872.63 (195.81)	(-1335.65, -409.62)				
	Week 22		5	5 (100.0)	-898.53 (220.89)	(-1439.04, -358.03)				
	Week 24		5	5 (100.0)	-671.54 (167.20)	(-1066.92, -276.16)				
	Week 26	5	3 (60.0)	-725.36 (216.32)	(-1281.42, -169.30)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 LDH = Lactate dehydrogenase levels (U/l).
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHC: Change from baseline in LDH (censored) by age
ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LDH level (censored) < 60	Baseline	Peg	29	29 (100.0)	2012.75 (709.15)	538.0	2061.29	3383.0	
		SoC	13	13 (100.0)	2116.46 (1101.43)	630.0	2032.50	4895.5	
	Week 2	Peg	29	28 (96.6)	238.50 (115.89)	129.0	206.00	753.0	
		SoC	13	12 (92.3)	2098.53 (1130.38)	438.0	1946.00	5008.0	
	Week 4	Peg	29	27 (93.1)	149.44 (50.11)	91.0	146.00	337.0	
		SoC	13	12 (92.3)	2197.46 (1245.59)	240.0	2235.50	4612.0	
	Week 6	Peg	29	27 (93.1)	152.25 (46.46)	102.0	144.00	330.0	
		SoC	13	13 (100.0)	2118.72 (1469.86)	606.0	1707.00	5582.0	
	Week 8	Peg	29	28 (96.6)	166.67 (55.90)	56.3	159.00	351.0	
		SoC	13	13 (100.0)	2188.41 (1545.05)	548.0	1770.18	5891.0	
	Week 10	Peg	29	28 (96.6)	174.78 (48.09)	58.2	168.50	309.0	
		SoC	13	13 (100.0)	2126.40 (1358.40)	664.0	1770.18	4899.0	
	Week 12	Peg	29	28 (96.6)	184.80 (39.93)	127.0	175.86	311.0	
		SoC	13	13 (100.0)	2067.27 (1291.05)	732.0	1707.00	4824.0	
	Week 14	Peg	29	28 (96.6)	189.79 (48.11)	128.0	178.64	351.0	
		SoC	13	13 (100.0)	2146.74 (1402.06)	592.0	1707.00	4964.5	
	Week 16	Peg	29	28 (96.6)	196.14 (75.10)	129.0	185.00	540.0	
		SoC	13	13 (100.0)	2103.71 (1436.46)	613.0	1707.00	5208.0	
	Week 18	Peg	29	27 (93.1)	211.71 (112.44)	122.0	182.00	700.0	
		SoC	13	13 (100.0)	2185.32 (1491.25)	719.0	1707.00	5544.0	
	Week 20	Peg	29	28 (96.6)	184.62 (62.75)	119.0	165.00	441.1	
		SoC	13	12 (92.3)	2439.32 (2280.80)	626.0	1661.00	8799.7	
	Week 22	Peg	29	28 (96.6)	189.80 (54.77)	124.0	173.00	343.0	
		SoC	13	12 (92.3)	2498.66 (2269.56)	789.0	1738.59	8799.7	
	Week 24	Peg	29	28 (96.6)	193.92 (67.11)	104.0	183.72	473.3	
		SoC	13	13 (100.0)	2274.59 (2254.02)	417.8	1707.00	8799.7	
	Week 26	Peg	29	24 (82.8)	212.85 (98.04)	122.0	183.00	612.0	
		SoC	13	11 (84.6)	2464.44 (2387.30)	626.0	1770.18	8799.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
LDH = Lactate dehydrogenase levels (U/l).
Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHC: Change from baseline in LDH (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LDH level (censored) >= 60	Baseline	Peg	6	6 (100.0)	2818.92 (1470.21)	1425.0	2351.50	4946.0	
		SoC	5	5 (100.0)	1502.60 (549.68)	826.0	1531.00	2041.0	
	Week 2	Peg	6	4 (66.7)	268.25 (47.84)	230.0	252.50	338.0	
		SoC	5	5 (100.0)	1380.60 (610.72)	674.0	1470.00	2086.0	
	Week 4	Peg	6	6 (100.0)	154.57 (17.05)	138.0	150.50	181.4	
		SoC	5	5 (100.0)	1455.80 (663.38)	615.0	1698.00	2062.0	
	Week 6	Peg	6	6 (100.0)	161.42 (45.78)	125.0	150.50	251.5	
		SoC	5	5 (100.0)	1379.00 (451.67)	845.0	1549.00	1952.0	
	Week 8	Peg	6	5 (83.3)	156.00 (7.97)	146.0	158.00	167.0	
		SoC	5	5 (100.0)	1488.40 (530.10)	909.0	1767.00	1955.0	
	Week 10	Peg	6	6 (100.0)	167.57 (7.61)	157.0	167.00	180.0	
		SoC	5	5 (100.0)	1435.60 (498.20)	909.0	1563.00	1955.0	
	Week 12	Peg	6	6 (100.0)	186.73 (43.28)	161.0	168.50	274.0	
		SoC	5	5 (100.0)	1490.60 (529.95)	909.0	1835.00	1955.0	
	Week 14	Peg	6	6 (100.0)	166.44 (24.61)	123.0	169.50	194.6	
		SoC	5	5 (100.0)	1375.20 (497.20)	909.0	1261.00	1955.0	
	Week 16	Peg	6	6 (100.0)	165.92 (23.12)	121.0	173.00	185.0	
		SoC	5	5 (100.0)	1362.00 (501.84)	909.0	1195.00	1955.0	
	Week 18	Peg	6	6 (100.0)	169.92 (27.40)	126.0	169.00	211.0	
		SoC	5	5 (100.0)	1481.20 (522.61)	909.0	1791.00	1955.0	
	Week 20	Peg	6	6 (100.0)	168.33 (37.59)	112.0	172.00	226.0	
		SoC	5	5 (100.0)	1311.60 (534.41)	909.0	943.00	1955.0	
	Week 22	Peg	6	5 (83.3)	175.53 (18.47)	158.0	168.00	204.7	
		SoC	5	5 (100.0)	1291.00 (553.80)	840.0	916.00	1955.0	
	Week 24	Peg	6	6 (100.0)	176.99 (33.65)	114.0	181.50	205.9	
		SoC	5	5 (100.0)	1472.40 (516.43)	909.0	1747.00	1955.0	
	Week 26	Peg	6	6 (100.0)	171.64 (33.78)	109.0	181.00	205.0	
		SoC	5	3 (60.0)	1566.33 (572.42)	909.0	1835.00	1955.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHC: Change from baseline in LDH (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in LDH (censored)	Week 2	Peg	29	28 (96.6)	-1768.24 (733.17)	-3117.0	-1820.75	-347.0	-2.42 (0.46) [-3.46, -1.66]
		SoC	13	12 (92.3)	-112.51 (484.76)	-1000.0	-2.50	775.3	
	Week 4	Peg	29	27 (93.1)	-1879.46 (723.12)	-3235.0	-1875.50	-374.0	-2.62 (0.48) [-3.71, -1.83]
		SoC	13	12 (92.3)	-13.58 (637.72)	-1043.0	-66.25	1282.0	
	Week 6	Peg	29	27 (93.1)	-1828.66 (716.95)	-3233.0	-1882.00	-373.0	-2.29 (0.44) [-3.28, -1.55]
		SoC	13	13 (100.0)	2.26 (916.17)	-1194.0	-31.30	2399.5	
	Week 8	Peg	29	28 (96.6)	-1840.07 (723.26)	-3219.0	-1872.64	-312.0	-2.38 (0.45) [-3.38, -1.63]
		SoC	13	13 (100.0)	71.94 (919.26)	-1043.0	-84.50	2399.5	
	Week 10	Peg	29	28 (96.6)	-1831.95 (717.71)	-3223.0	-1892.87	-291.2	-2.36 (0.44) [-3.36, -1.61]
		SoC	13	13 (100.0)	9.94 (867.23)	-1043.0	-84.50	2399.5	
	Week 12	Peg	29	28 (96.6)	-1821.94 (720.11)	-3218.0	-1875.17	-377.0	-2.25 (0.44) [-3.23, -1.52]
		SoC	13	13 (100.0)	-49.19 (878.76)	-1043.0	-84.50	2399.5	
	Week 14	Peg	29	28 (96.6)	-1816.95 (725.92)	-3209.0	-1884.53	-381.0	-2.35 (0.44) [-3.35, -1.61]
		SoC	13	13 (100.0)	30.28 (864.89)	-1043.0	-84.50	2399.5	
	Week 16	Peg	29	28 (96.6)	-1810.60 (725.51)	-3202.0	-1860.12	-401.4	-2.31 (0.44) [-3.30, -1.57]
		SoC	13	13 (100.0)	-12.76 (844.43)	-1043.0	-17.00	2399.5	
	Week 18	Peg	29	27 (93.1)	-1781.19 (726.05)	-3214.0	-1824.00	-393.0	-2.31 (0.45) [-3.32, -1.57]
		SoC	13	13 (100.0)	68.86 (897.73)	-1043.0	-58.50	2399.5	
	Week 20	Peg	29	28 (96.6)	-1822.12 (713.64)	-3218.0	-1893.25	-413.0	-2.02 (0.43) [-2.99, -1.30]
		SoC	13	12 (92.3)	228.28 (1467.77)	-1043.0	-82.25	3904.2	
Week 22	Peg	29	28 (96.6)	-1816.93 (735.66)	-3207.0	-1892.25	-195.0	-2.07 (0.43) [-3.04, -1.33]	
	SoC	13	12 (92.3)	287.61 (1453.59)	-1043.0	4.50	3904.2		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHC: Change from baseline in LDH (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in LDH (censored)	Week 24	Peg	29	28 (96.6)	-1812.81 (714.42)	-3199.0	-1866.14	-434.0	-1.90 (0.41) [-2.82, -1.20]
		SoC	13	13 (100.0)	158.13 (1485.13)	-1614.7	-84.50	3904.2	
	Week 26	Peg	29	24 (82.8)	-1909.55 (707.20)	-3221.0	-1996.00	-675.0	-2.27 (0.48) [-3.36, -1.48]
		SoC	13	11 (84.6)	389.58 (1441.64)	-1026.0	-84.50	3904.2	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHC: Change from baseline in LDH (censored) by age
ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in LDH (censored)	Week 2	Peg	6	4 (66.7)	-2784.38 (1807.02)	-4689.0	-2626.75	-1195.0	-1.99 (1.08) [-4.82, -0.66]
		SoC	5	5 (100.0)	-122.00 (118.51)	-236.0	-152.00	52.0	
	Week 4	Peg	6	6 (100.0)	-2664.34 (1475.28)	-4808.0	-2176.78	-1286.0	-2.13 (0.94) [-4.54, -0.90]
		SoC	5	5 (100.0)	-46.80 (342.56)	-343.0	-189.00	531.0	
	Week 6	Peg	6	6 (100.0)	-2657.50 (1485.90)	-4821.0	-2158.74	-1268.0	-2.07 (0.92) [-4.43, -0.84]
		SoC	5	5 (100.0)	-123.60 (253.66)	-491.0	-82.00	173.0	
	Week 8	Peg	6	5 (83.3)	-2786.40 (1611.42)	-4800.0	-2334.50	-1267.0	-2.19 (1.02) [-4.85, -0.90]
		SoC	5	5 (100.0)	-14.20 (169.94)	-165.0	-86.00	236.0	
	Week 10	Peg	6	6 (100.0)	-2651.35 (1470.67)	-4789.0	-2184.29	-1257.0	-2.12 (0.94) [-4.52, -0.89]
		SoC	5	5 (100.0)	-67.00 (288.67)	-471.0	-86.00	304.0	
	Week 12	Peg	6	6 (100.0)	-2632.19 (1478.43)	-4779.0	-2123.82	-1255.0	-2.16 (0.94) [-4.58, -0.92]
		SoC	5	5 (100.0)	-12.00 (207.14)	-196.0	-86.00	304.0	
	Week 14	Peg	6	6 (100.0)	-2652.48 (1471.28)	-4787.0	-2205.50	-1255.0	-2.04 (0.92) [-4.40, -0.82]
		SoC	5	5 (100.0)	-127.40 (403.21)	-773.0	-86.00	304.0	
	Week 16	Peg	6	6 (100.0)	-2652.99 (1469.90)	-4782.0	-2198.50	-1253.0	-2.03 (0.92) [-4.37, -0.81]
		SoC	5	5 (100.0)	-140.60 (429.83)	-839.0	-86.00	304.0	
	Week 18	Peg	6	6 (100.0)	-2648.99 (1478.53)	-4781.0	-2202.50	-1214.0	-2.16 (0.94) [-4.59, -0.92]
		SoC	5	5 (100.0)	-21.40 (218.34)	-243.0	-86.00	304.0	
	Week 20	Peg	6	6 (100.0)	-2650.58 (1479.37)	-4769.0	-2212.00	-1246.0	-1.94 (0.90) [-4.23, -0.73]
		SoC	5	5 (100.0)	-191.00 (534.28)	-1091.0	-86.00	304.0	
	Week 22	Peg	6	5 (83.3)	-2766.87 (1616.13)	-4788.0	-2333.50	-1260.0	-1.90 (0.96) [-4.37, -0.64]
		SoC	5	5 (100.0)	-211.60 (577.86)	-1194.0	-86.00	304.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
LDH = Lactate dehydrogenase levels (U/l).
Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHC: Change from baseline in LDH (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in LDH (censored)	Week 24	Peg	6	6 (100.0)	-2641.93 (1461.22)	-4758.0	-2207.00	-1250.0	-2.17 (0.94) [-4.60, -0.93]
		SoC	5	5 (100.0)	-30.20 (230.07)	-287.0	-86.00	304.0	
	Week 26	Peg	6	6 (100.0)	-2647.28 (1463.02)	-4770.0	-2215.50	-1239.0	-1.97 (1.10) [-4.84, -0.57]
		SoC	5	3 (60.0)	100.33 (195.58)	-86.0	83.00	304.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SICD: Change from baseline in LDH (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.3844

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 LDH = Lactate dehydrogenase levels (U/l).
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SICD: Change from baseline in LDH (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Male	Peg	Week 2	19	18 (94.7)	-1984.43 (89.82)	(-2170.24, -1798.63)	-1687.60 (161.23)	(-2021.14, -1354.07)	<0.0001	*
		Week 4	19	18 (94.7)	-2078.65 (75.61)	(-2235.05, -1922.24)	-1757.69 (136.82)	(-2040.73, -1474.66)	<0.0001	*
		Week 6	19	18 (94.7)	-1967.03 (64.74)	(-2100.64, -1833.42)	-1465.63 (111.57)	(-1695.90, -1235.37)	<0.0001	*
		Week 8	19	18 (94.7)	-1984.69 (79.65)	(-2149.09, -1820.30)	-1600.89 (140.13)	(-1890.11, -1311.66)	<0.0001	*
		Week 10	19	19 (100.0)	-1976.99 (67.02)	(-2115.03, -1838.96)	-1533.36 (119.06)	(-1778.57, -1288.15)	<0.0001	*
		Week 12	19	19 (100.0)	-1977.77 (60.27)	(-2101.90, -1853.64)	-1519.42 (107.07)	(-1739.93, -1298.91)	<0.0001	*
		Week 14	19	19 (100.0)	-1970.54 (81.13)	(-2137.63, -1803.45)	-1517.37 (144.12)	(-1814.19, -1220.54)	<0.0001	*
		Week 16	19	19 (100.0)	-1984.10 (86.60)	(-2162.46, -1805.75)	-1520.08 (153.84)	(-1836.91, -1203.24)	<0.0001	*
		Week 18	19	19 (100.0)	-1948.85 (76.74)	(-2106.89, -1790.81)	-1517.74 (136.32)	(-1798.49, -1236.99)	<0.0001	*
		Week 20	19	19 (100.0)	-2005.33 (83.96)	(-2178.62, -1832.04)	-1529.55 (152.35)	(-1843.98, -1215.11)	<0.0001	*
		Week 22	19	18 (94.7)	-2001.66 (98.41)	(-2205.23, -1798.08)	-1545.14 (176.66)	(-1910.58, -1179.70)	<0.0001	*
		Week 24	19	19 (100.0)	-1967.58 (78.79)	(-2129.85, -1805.32)	-1381.82 (139.96)	(-1670.06, -1093.57)	<0.0001	*
		Week 26	19	17 (89.5)	-2084.31 (77.20)	(-2244.86, -1923.77)	-1545.34 (144.54)	(-1845.93, -1244.74)	<0.0001	*
		SoC	Week 2	10	9 (90.0)	-296.83 (132.40)	(-570.72, -22.94)			
	Week 4		10	9 (90.0)	-320.95 (112.18)	(-553.01, -88.89)				
	Week 6		10	10 (100.0)	-501.39 (91.15)	(-689.51, -313.27)				
	Week 8		10	10 (100.0)	-383.80 (113.10)	(-617.23, -150.38)				
	Week 10		10	10 (100.0)	-443.63 (97.41)	(-644.25, -243.01)				
	Week 12		10	10 (100.0)	-458.35 (87.60)	(-638.75, -277.94)				
	Week 14		10	10 (100.0)	-453.17 (117.91)	(-696.01, -210.33)				
	Week 16		10	10 (100.0)	-464.03 (125.86)	(-723.24, -204.81)				
	Week 18		10	10 (100.0)	-431.11 (111.53)	(-660.80, -201.41)				
	Week 20		10	9 (90.0)	-475.78 (126.65)	(-737.18, -214.39)				
	Week 22		10	9 (90.0)	-456.52 (145.06)	(-756.60, -156.43)				
	Week 24		10	10 (100.0)	-585.77 (114.51)	(-821.60, -349.94)				
	Week 26	10	8 (80.0)	-538.98 (117.82)	(-784.01, -293.95)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 LDH = Lactate dehydrogenase levels (U/l).
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SICD: Change from baseline in LDH (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA				
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA	
Female	Peg	Week 2	16	14 (87.5)	-1620.31 (139.41)	(-1913.21, -1327.41)	-1448.30 (255.07)	(-1984.18, -912.42)	<0.0001	*	
		Week 4	16	15 (93.8)	-1765.26 (159.17)	(-2098.41, -1432.10)	-1766.71 (298.77)	(-2392.04, -1141.37)	<0.0001	*	
		Week 6	16	15 (93.8)	-1761.40 (207.39)	(-2195.47, -1327.34)	-1957.53 (389.27)	(-2772.27, -1142.79)	<0.0001	*	
		Week 8	16	15 (93.8)	-1747.77 (212.13)	(-2191.77, -1303.77)	-1977.45 (398.18)	(-2810.84, -1144.05)	<0.0001	*	
		Week 10	16	15 (93.8)	-1751.59 (200.72)	(-2171.70, -1331.48)	-1921.71 (376.75)	(-2710.25, -1133.17)	<0.0001	*	
		Week 12	16	15 (93.8)	-1731.06 (197.55)	(-2144.54, -1317.57)	-1859.45 (370.81)	(-2635.56, -1083.34)	<0.0001	*	
		Week 14	16	15 (93.8)	-1735.88 (205.43)	(-2165.86, -1305.90)	-1930.21 (385.60)	(-2737.28, -1123.14)	<0.0001	*	
		Week 16	16	15 (93.8)	-1711.75 (203.19)	(-2137.04, -1286.46)	-1860.05 (381.40)	(-2658.33, -1061.78)	0.0001	*	
		Week 18	16	14 (87.5)	-1673.81 (229.26)	(-2155.46, -1192.16)	-1899.56 (425.71)	(-2793.95, -1005.17)	0.0003	*	
		Week 20	16	15 (93.8)	-1686.19 (294.67)	(-2302.94, -1069.45)	-2073.40 (553.09)	(-3231.04, -915.76)	0.0014	*	
		Week 22	16	15 (93.8)	-1677.51 (291.41)	(-2287.44, -1067.58)	-2106.82 (546.98)	(-3251.66, -961.98)	0.0011	*	
		Week 24	16	15 (93.8)	-1651.91 (285.46)	(-2249.39, -1054.42)	-2152.37 (535.82)	(-3273.85, -1030.89)	0.0007	*	
		Week 26	16	13 (81.3)	-1732.52 (309.30)	(-2391.78, -1073.27)	-2854.75 (656.39)	(-4253.81, -1455.69)	0.0006	*	
			SoC	Week 2	8	8 (100.0)	-172.01 (204.88)	(-602.44, 258.42)			
		Week 4		8	8 (100.0)	1.45 (239.63)	(-500.11, 503.01)				
		Week 6		8	8 (100.0)	196.13 (312.22)	(-457.35, 849.61)				
		Week 8		8	8 (100.0)	229.68 (319.37)	(-438.76, 898.12)				
		Week 10		8	8 (100.0)	170.12 (302.18)	(-462.35, 802.59)				
		Week 12		8	8 (100.0)	128.39 (297.41)	(-494.10, 750.89)				
		Week 14		8	8 (100.0)	194.33 (309.28)	(-453.00, 841.65)				
		Week 16		8	8 (100.0)	148.31 (305.91)	(-491.97, 788.58)				
		Week 18		8	8 (100.0)	225.75 (331.15)	(-469.97, 921.46)				
		Week 20		8	8 (100.0)	387.21 (443.62)	(-541.30, 1315.71)				
		Week 22		8	8 (100.0)	429.32 (438.71)	(-488.92, 1347.56)				
		Week 24		8	8 (100.0)	500.46 (429.76)	(-399.04, 1399.97)				
		Week 26	8	6 (75.0)	1122.23 (539.97)	(-28.68, 2273.14)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 LDH = Lactate dehydrogenase levels (U/l).
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHD: Change from baseline in LDH (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LDH level (censored)	Male	Baseline	Peg	19	19 (100.0)	2356.01 (1111.98)	538.0	2260.50	4946.0	
			SoC	10	10 (100.0)	1743.80 (622.51)	630.0	1951.25	2835.0	
		Week 2	Peg	19	18 (94.7)	226.10 (65.31)	129.0	208.50	352.0	
			SoC	10	9 (90.0)	1842.59 (675.28)	438.0	1865.00	2758.3	
		Week 4	Peg	19	18 (94.7)	150.13 (35.64)	99.0	148.50	269.0	
			SoC	10	9 (90.0)	1838.72 (559.86)	591.0	2012.00	2355.5	
		Week 6	Peg	19	18 (94.7)	160.41 (37.61)	120.0	153.00	252.5	
			SoC	10	10 (100.0)	1559.64 (466.24)	606.0	1595.50	2178.0	
		Week 8	Peg	19	18 (94.7)	172.96 (36.86)	120.0	165.00	268.6	
			SoC	10	10 (100.0)	1675.03 (583.40)	548.0	1832.59	2520.0	
		Week 10	Peg	19	19 (100.0)	179.95 (28.60)	136.0	170.42	246.8	
			SoC	10	10 (100.0)	1629.92 (513.90)	664.0	1802.59	2197.0	
		Week 12	Peg	19	19 (100.0)	179.32 (24.02)	148.0	167.00	240.2	
			SoC	10	10 (100.0)	1624.04 (461.88)	732.0	1802.59	2187.0	
		Week 14	Peg	19	19 (100.0)	189.23 (48.60)	123.0	178.29	351.0	
			SoC	10	10 (100.0)	1606.72 (619.16)	592.0	1713.09	2700.0	
		Week 16	Peg	19	19 (100.0)	177.79 (27.75)	121.0	179.54	221.0	
			SoC	10	10 (100.0)	1595.52 (659.97)	613.0	1657.09	2913.0	
		Week 18	Peg	19	19 (100.0)	213.98 (124.22)	126.0	179.54	700.0	
			SoC	10	10 (100.0)	1650.72 (551.95)	719.0	1780.59	2631.0	
		Week 20	Peg	19	19 (100.0)	188.65 (69.57)	112.0	165.00	441.1	
			SoC	10	9 (90.0)	1651.35 (645.09)	626.0	1770.18	2755.0	
		Week 22	Peg	19	18 (94.7)	194.06 (48.39)	152.0	176.00	343.0	
			SoC	10	9 (90.0)	1674.35 (717.34)	811.0	1770.18	3123.0	
		Week 24	Peg	19	19 (100.0)	183.66 (35.54)	104.0	184.00	241.1	
			SoC	10	10 (100.0)	1483.40 (593.56)	417.8	1758.59	2035.0	
		Week 26	Peg	19	17 (89.5)	188.36 (38.59)	109.0	186.00	270.0	
			SoC	10	8 (80.0)	1610.52 (586.30)	626.0	1802.59	2314.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHD: Change from baseline in LDH (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LDH level (censored)	Female	Baseline	Peg	16	16 (100.0)	1907.44 (523.76)	1121.5	1944.25	2698.5	
			SoC	8	8 (100.0)	2198.63 (1348.02)	826.0	2261.00	4895.5	
		Week 2	Peg	16	14 (87.5)	262.93 (149.20)	165.0	220.50	753.0	
			SoC	8	8 (100.0)	1937.75 (1397.95)	674.0	1700.50	5008.0	
		Week 4	Peg	16	15 (93.8)	150.67 (56.87)	91.0	146.00	337.0	
			SoC	8	8 (100.0)	2137.50 (1598.32)	240.0	1952.00	4612.0	
		Week 6	Peg	16	15 (93.8)	146.13 (54.31)	102.0	129.00	330.0	
			SoC	8	8 (100.0)	2355.25 (1849.64)	824.0	1661.00	5582.0	
		Week 8	Peg	16	15 (93.8)	155.56 (65.20)	56.3	143.00	351.0	
			SoC	8	8 (100.0)	2392.63 (1934.19)	789.0	1661.00	5891.0	
		Week 10	Peg	16	15 (93.8)	165.36 (57.62)	58.2	152.24	309.0	
			SoC	8	8 (100.0)	2315.25 (1706.91)	789.0	1661.00	4899.0	
		Week 12	Peg	16	15 (93.8)	192.50 (53.84)	127.0	178.72	311.0	
			SoC	8	8 (100.0)	2260.89 (1630.22)	789.0	1661.00	4824.0	
		Week 14	Peg	16	15 (93.8)	181.16 (42.40)	128.0	169.00	275.0	
			SoC	8	8 (100.0)	2339.56 (1727.53)	789.0	1661.00	4964.5	
		Week 16	Peg	16	15 (93.8)	207.29 (99.39)	129.0	186.00	540.0	
			SoC	8	8 (100.0)	2275.38 (1763.95)	789.0	1661.00	5208.0	
		Week 18	Peg	16	14 (87.5)	190.71 (67.31)	122.0	177.00	368.0	
			SoC	8	8 (100.0)	2413.50 (1860.91)	789.0	1661.00	5544.0	
		Week 20	Peg	16	15 (93.8)	173.00 (42.71)	119.0	165.00	260.0	
			SoC	8	8 (100.0)	2620.96 (2816.85)	789.0	1511.50	8799.7	
		Week 22	Peg	16	15 (93.8)	179.93 (54.62)	124.0	168.00	313.0	
			SoC	8	8 (100.0)	2671.21 (2795.63)	789.0	1661.00	8799.7	
		Week 24	Peg	16	15 (93.8)	200.15 (86.06)	130.0	175.00	473.3	
			SoC	8	8 (100.0)	2762.21 (2775.36)	789.0	1661.00	8799.7	
		Week 26	Peg	16	13 (81.3)	225.85 (129.34)	122.0	173.00	612.0	
			SoC	8	6 (75.0)	3153.95 (3129.69)	789.0	1801.00	8799.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHHD: Change from baseline in LDH (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in LDH (censored)	Male	Week 2	Peg	19	18 (94.7)	-2138.49 (1116.54)	-4689.0	-2119.42	-347.0	-2.23 (0.55) [-3.49, -1.35]
			SoC	10	9 (90.0)	14.09 (311.30)	-273.0	-54.50	775.3	
	Week 4	Peg	19	18 (94.7)	-2258.52 (1125.08)	-4808.0	-2203.80	-374.0	-2.31 (0.55) [-3.60, -1.43]	
		SoC	10	9 (90.0)	10.22 (373.93)	-653.0	-38.50	531.0		
	Week 6	Peg	19	18 (94.7)	-2176.26 (1149.63)	-4821.0	-2045.24	-373.0	-2.01 (0.51) [-3.19, -1.19]	
		SoC	10	10 (100.0)	-184.16 (406.64)	-1194.0	-56.65	258.5		
	Week 8	Peg	19	18 (94.7)	-2191.63 (1160.53)	-4800.0	-2180.06	-312.0	-2.14 (0.52) [-3.34, -1.30]	
		SoC	10	10 (100.0)	-68.77 (364.00)	-836.0	-89.75	600.5		
	Week 10	Peg	19	19 (100.0)	-2176.06 (1121.51)	-4789.0	-2124.50	-291.2	-2.15 (0.51) [-3.34, -1.32]	
		SoC	10	10 (100.0)	-113.88 (284.24)	-638.0	-112.25	304.0		
	Week 12	Peg	19	19 (100.0)	-2176.68 (1115.73)	-4779.0	-2112.50	-377.0	-2.16 (0.51) [-3.34, -1.32]	
		SoC	10	10 (100.0)	-119.76 (299.37)	-648.0	-54.13	304.0		
	Week 14	Peg	19	19 (100.0)	-2166.78 (1122.45)	-4787.0	-2078.50	-381.0	-2.12 (0.51) [-3.29, -1.29]	
		SoC	10	10 (100.0)	-137.08 (295.81)	-773.0	-110.50	304.0		
	Week 16	Peg	19	19 (100.0)	-2178.22 (1115.71)	-4782.0	-2080.50	-401.4	-2.11 (0.51) [-3.29, -1.29]	
		SoC	10	10 (100.0)	-148.28 (352.92)	-839.0	-51.50	304.0		
	Week 18	Peg	19	19 (100.0)	-2142.03 (1121.43)	-4781.0	-2064.50	-393.0	-2.14 (0.51) [-3.33, -1.31]	
		SoC	10	10 (100.0)	-93.08 (270.27)	-687.5	-72.25	304.0		
	Week 20	Peg	19	19 (100.0)	-2167.36 (1115.16)	-4769.0	-2089.50	-413.0	-2.01 (0.52) [-3.19, -1.16]	
		SoC	10	9 (90.0)	-177.15 (461.96)	-1091.0	-80.00	304.0		
Week 22	Peg	19	18 (94.7)	-2170.53 (1163.68)	-4788.0	-2102.92	-195.0	-1.94 (0.52) [-3.13, -1.10]		
	SoC	10	9 (90.0)	-154.15 (538.04)	-1194.0	93.50	304.0			

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHD: Change from baseline in LDH (censored) by sex
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Male in LDH (censored)	Week 24	Peg	19	19 (100.0)	-2172.35 (1106.27)	-4758.0	-2110.50	-434.0	-1.93 (0.49) [-3.06, -1.12]
		SoC	10	10 (100.0)	-260.40 (572.96)	-1614.7	-106.75	304.0	
	Week 26	Peg	19	17 (89.5)	-2330.32 (1051.27)	-4770.0	-2157.68	-675.0	-2.47 (0.60) [-3.89, -1.54]
		SoC	10	8 (80.0)	-60.92 (245.44)	-521.0	-45.00	304.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHD: Change from baseline in LDH (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in LDH (censored)	Female	Week 2	Peg	16	14 (87.5)	-1582.54 (574.85)	-2368.5	-1622.00	-636.0	-2.35 (0.62) [-3.84, -1.40]
			SoC	8	8 (100.0)	-260.88 (469.94)	-1000.0	-75.75	127.5	
	Week 4	Peg	16	15 (93.8)	-1738.53 (518.99)	-2409.0	-1792.50	-969.5	-2.69 (0.65) [-4.24, -1.71]	
		SoC	8	8 (100.0)	-61.13 (737.44)	-1043.0	-200.00	1282.0		
	Week 6	Peg	16	15 (93.8)	-1743.07 (519.14)	-2409.0	-1800.50	-993.5	-2.41 (0.62) [-3.87, -1.46]	
		SoC	8	8 (100.0)	156.63 (1094.32)	-1043.0	61.75	2399.5		
	Week 8	Peg	16	15 (93.8)	-1733.64 (513.92)	-2392.0	-1792.50	-978.5	-2.41 (0.62) [-3.87, -1.46]	
		SoC	8	8 (100.0)	194.00 (1120.16)	-1043.0	-0.75	2399.5		
	Week 10	Peg	16	15 (93.8)	-1723.84 (513.08)	-2389.5	-1768.50	-969.5	-2.34 (0.61) [-3.78, -1.40]	
		SoC	8	8 (100.0)	116.63 (1096.53)	-1043.0	-40.50	2399.5		
	Week 12	Peg	16	15 (93.8)	-1696.70 (523.41)	-2387.5	-1742.50	-958.5	-2.21 (0.59) [-3.61, -1.28]	
		SoC	8	8 (100.0)	62.27 (1101.26)	-1043.0	-124.75	2399.5		
	Week 14	Peg	16	15 (93.8)	-1708.04 (538.43)	-2423.5	-1718.50	-953.5	-2.30 (0.60) [-3.72, -1.36]	
		SoC	8	8 (100.0)	140.93 (1107.47)	-1043.0	-7.77	2399.5		
	Week 16	Peg	16	15 (93.8)	-1681.91 (537.86)	-2424.5	-1595.50	-937.5	-2.24 (0.60) [-3.64, -1.31]	
		SoC	8	8 (100.0)	76.75 (1069.63)	-1043.0	-0.75	2399.5		
	Week 18	Peg	16	14 (87.5)	-1663.39 (544.17)	-2423.5	-1652.00	-939.5	-2.27 (0.61) [-3.73, -1.32]	
		SoC	8	8 (100.0)	214.88 (1122.01)	-1043.0	-0.75	2399.5		
	Week 20	Peg	16	15 (93.8)	-1716.20 (534.12)	-2438.5	-1752.50	-956.5	-1.84 (0.56) [-3.14, -0.95]	
		SoC	8	8 (100.0)	422.34 (1782.08)	-1043.0	-124.75	3904.2		
	Week 22	Peg	16	15 (93.8)	-1709.27 (542.92)	-2456.5	-1791.50	-953.5	-1.90 (0.56) [-3.21, -1.01]	
		SoC	8	8 (100.0)	472.59 (1751.66)	-1043.0	-124.75	3904.2		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHD: Change from baseline in LDH (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in LDH (censored)	Female	Week 24	Peg	16	15 (93.8)	-1689.05 (525.53)	-2472.5	-1778.50	-956.5	-2.00 (0.57) [-3.34, -1.09]
			SoC	8	8 (100.0)	563.59 (1725.24)	-1043.0	-0.75	3904.2	
		Week 26	Peg	16	13 (81.3)	-1699.81 (592.06)	-2416.5	-1801.00	-803.0	-2.14 (0.67) [-3.73, -1.11]
			SoC	8	6 (75.0)	845.62 (1887.73)	-1026.0	-0.75	3904.2	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SICB: Change from baseline in LDH (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.7906

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 LDH = Lactate dehydrogenase levels (U/l).
 Source Data: aeфф, created on: 13SEP2023

Table PT1LDHWC_SICB: Change from baseline in LDH (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Latin America	Peg	Week 2	12	10 (83.3)	-2003.04 (92.78)	(-2216.99, -1789.09)	-1933.29 (233.35)	(-2471.41, -1395.18)	<0.0001	*
		Week 4	12	11 (91.7)	-2084.09 (72.65)	(-2248.42, -1919.75)	-1691.22 (189.86)	(-2120.71, -1261.73)	<0.0001	*
		Week 6	12	11 (91.7)	-2081.74 (26.23)	(-2141.09, -2022.40)	-1497.54 (68.56)	(-1652.63, -1342.45)	<0.0001	*
		Week 8	12	11 (91.7)	-2053.43 (26.60)	(-2113.60, -1993.27)	-1463.90 (69.51)	(-1621.14, -1306.66)	<0.0001	*
		Week 10	12	11 (91.7)	-2051.00 (22.35)	(-2101.56, -2000.43)	-1463.05 (58.42)	(-1595.20, -1330.90)	<0.0001	*
		Week 12	12	11 (91.7)	-2048.04 (22.72)	(-2099.43, -1996.64)	-1464.09 (59.38)	(-1598.41, -1329.77)	<0.0001	*
		Week 14	12	11 (91.7)	-2042.73 (26.15)	(-2101.89, -1983.58)	-1452.12 (68.34)	(-1606.71, -1297.54)	<0.0001	*
		Week 16	12	11 (91.7)	-2063.07 (20.22)	(-2108.81, -2017.33)	-1477.96 (52.84)	(-1597.49, -1358.43)	<0.0001	*
		Week 18	12	10 (83.3)	-1998.44 (56.78)	(-2129.38, -1867.51)	-1416.74 (140.42)	(-1740.55, -1092.92)	<0.0001	*
		Week 20	12	11 (91.7)	-2049.36 (31.11)	(-2119.73, -1978.98)	-1462.94 (81.30)	(-1646.86, -1279.02)	<0.0001	*
		Week 22	12	11 (91.7)	-2041.24 (24.30)	(-2096.22, -1986.27)	-1440.65 (63.51)	(-1584.33, -1296.97)	<0.0001	*
		Week 24	12	11 (91.7)	-2032.53 (32.97)	(-2107.12, -1957.94)	-1458.70 (86.17)	(-1653.64, -1263.77)	<0.0001	*
		Week 26	12	10 (83.3)	-2174.01 (26.15)	(-2234.31, -2113.71)	-1444.28 (66.69)	(-1598.06, -1290.51)	<0.0001	*
	SoC	Week 2	2	2 (100.0)	-69.75 (211.92)	(-558.44, 418.95)				
		Week 4	2	2 (100.0)	-392.87 (174.43)	(-787.46, 1.72)				
		Week 6	2	2 (100.0)	-584.20 (62.99)	(-726.69, -441.72)				
		Week 8	2	2 (100.0)	-589.54 (63.86)	(-734.00, -445.07)				
		Week 10	2	2 (100.0)	-587.94 (53.67)	(-709.36, -466.53)				
		Week 12	2	2 (100.0)	-583.95 (54.55)	(-707.35, -460.54)				
Week 14		2	2 (100.0)	-590.61 (62.78)	(-732.64, -448.58)					
Week 16	2	2 (100.0)	-585.11 (48.55)	(-694.93, -475.29)						
Week 18	2	2 (100.0)	-581.71 (128.46)	(-877.93, -285.49)						
Week 20	2	2 (100.0)	-586.42 (74.70)	(-755.39, -417.44)						
Week 22	2	2 (100.0)	-600.60 (58.35)	(-732.60, -468.59)						
Week 24	2	2 (100.0)	-573.83 (79.17)	(-752.92, -394.73)						
Week 26	2	2 (100.0)	-729.73 (60.70)	(-869.69, -589.76)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 LDH = Lactate dehydrogenase levels (U/l).
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SICB: Change from baseline in LDH (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Asia Pacific Peg		Week 2	23	22 (95.7)	-1796.79 (113.89)	(-2028.51, -1565.07)	-1603.29 (180.31)	(-1970.13, -1236.45)	<0.0001	*
		Week 4	23	22 (95.7)	-1923.43 (130.39)	(-2188.70, -1658.16)	-1850.90 (206.48)	(-2270.98, -1430.83)	<0.0001	*
		Week 6	23	22 (95.7)	-1832.34 (159.43)	(-2156.34, -1508.34)	-1771.63 (245.24)	(-2270.02, -1273.23)	<0.0001	*
		Week 8	23	22 (95.7)	-1865.10 (163.76)	(-2197.91, -1532.29)	-1893.17 (253.08)	(-2407.49, -1378.85)	<0.0001	*
		Week 10	23	23 (100.0)	-1854.23 (143.73)	(-2146.03, -1562.43)	-1810.52 (224.68)	(-2266.64, -1354.39)	<0.0001	*
		Week 12	23	23 (100.0)	-1839.28 (139.19)	(-2121.84, -1556.72)	-1763.26 (217.57)	(-2204.95, -1321.58)	<0.0001	*
		Week 14	23	23 (100.0)	-1838.60 (148.85)	(-2140.78, -1536.43)	-1792.20 (232.67)	(-2264.54, -1319.86)	<0.0001	*
		Week 16	23	23 (100.0)	-1828.19 (151.81)	(-2136.39, -1520.00)	-1746.85 (237.30)	(-2228.60, -1265.10)	<0.0001	*
		Week 18	23	23 (100.0)	-1831.87 (155.64)	(-2147.83, -1515.91)	-1851.69 (243.28)	(-2345.58, -1357.80)	<0.0001	*
		Week 20	23	23 (100.0)	-1853.27 (228.16)	(-2316.95, -1389.60)	-1950.40 (365.61)	(-2693.40, -1207.40)	<0.0001	*
		Week 22	23	22 (95.7)	-1846.26 (235.58)	(-2325.56, -1366.96)	-1985.45 (372.96)	(-2744.23, -1226.66)	<0.0001	*
		Week 24	23	23 (100.0)	-1829.25 (226.14)	(-2288.34, -1370.16)	-1921.78 (353.49)	(-2639.41, -1204.16)	<0.0001	*
		Week 26	23	20 (87.0)	-1906.67 (254.80)	(-2428.61, -1384.73)	-2257.50 (427.22)	(-3132.63, -1382.38)	<0.0001	*
SoC		Week 2	16	15 (93.8)	-193.50 (137.69)	(-473.64, 86.63)				
		Week 4	16	15 (93.8)	-72.52 (157.64)	(-393.24, 248.20)				
		Week 6	16	16 (100.0)	-60.72 (183.41)	(-433.46, 312.03)				
		Week 8	16	16 (100.0)	28.07 (190.79)	(-359.66, 415.80)				
		Week 10	16	16 (100.0)	-43.72 (170.42)	(-389.68, 302.25)				
		Week 12	16	16 (100.0)	-76.02 (165.02)	(-411.04, 258.99)				
		Week 14	16	16 (100.0)	-46.40 (176.48)	(-404.67, 311.86)				
		Week 16	16	16 (100.0)	-81.35 (179.99)	(-446.75, 284.06)				
		Week 18	16	16 (100.0)	19.82 (184.53)	(-354.79, 394.43)				
		Week 20	16	15 (93.8)	97.12 (280.66)	(-473.25, 667.50)				
		Week 22	16	15 (93.8)	139.19 (284.80)	(-440.25, 718.62)				
		Week 24	16	16 (100.0)	92.53 (268.12)	(-451.78, 636.85)				
		Week 26	16	12 (75.0)	350.83 (332.50)	(-330.26, 1031.92)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 LDH = Lactate dehydrogenase levels (U/l).
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHB: Change from baseline in LDH (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LDH level (censored)	Latin America	Baseline	Peg	12	12 (100.0)	2328.76 (1054.45)	538.0	2276.67	4946.0	
			SoC	2	2 (100.0)	1716.75 (376.53)	1450.5	1716.75	1983.0	
		Week 2	Peg	12	10 (83.3)	221.89 (64.76)	157.0	197.50	332.0	
			SoC	2	2 (100.0)	2153.67 (855.13)	1549.0	2153.67	2758.3	
		Week 4	Peg	12	11 (91.7)	166.19 (72.34)	91.0	141.00	337.0	
			SoC	2	2 (100.0)	1856.25 (706.05)	1357.0	1856.25	2355.5	
		Week 6	Peg	12	11 (91.7)	163.61 (66.67)	114.0	135.00	330.0	
			SoC	2	2 (100.0)	1657.09 (159.93)	1544.0	1657.09	1770.2	
		Week 8	Peg	12	11 (91.7)	191.48 (67.29)	117.3	167.00	351.0	
			SoC	2	2 (100.0)	1657.09 (159.93)	1544.0	1657.09	1770.2	
		Week 10	Peg	12	11 (91.7)	194.22 (49.53)	152.2	177.00	309.0	
			SoC	2	2 (100.0)	1657.09 (159.93)	1544.0	1657.09	1770.2	
		Week 12	Peg	12	11 (91.7)	199.21 (51.74)	161.0	178.72	311.0	
			SoC	2	2 (100.0)	1657.09 (159.93)	1544.0	1657.09	1770.2	
		Week 14	Peg	12	11 (91.7)	202.20 (65.33)	150.8	166.90	351.0	
			SoC	2	2 (100.0)	1657.09 (159.93)	1544.0	1657.09	1770.2	
		Week 16	Peg	12	11 (91.7)	183.73 (39.08)	136.6	175.88	274.0	
			SoC	2	2 (100.0)	1657.09 (159.93)	1544.0	1657.09	1770.2	
		Week 18	Peg	12	10 (83.3)	246.31 (167.72)	145.0	178.43	700.0	
			SoC	2	2 (100.0)	1657.09 (159.93)	1544.0	1657.09	1770.2	
		Week 20	Peg	12	11 (91.7)	199.40 (89.17)	125.0	167.00	441.1	
			SoC	2	2 (100.0)	1657.09 (159.93)	1544.0	1657.09	1770.2	
		Week 22	Peg	12	11 (91.7)	203.13 (61.00)	152.0	173.99	343.0	
			SoC	2	2 (100.0)	1657.09 (159.93)	1544.0	1657.09	1770.2	
		Week 24	Peg	12	11 (91.7)	214.71 (94.19)	104.0	188.00	473.3	
			SoC	2	2 (100.0)	1657.09 (159.93)	1544.0	1657.09	1770.2	
		Week 26	Peg	12	10 (83.3)	214.23 (54.82)	161.0	195.33	307.0	
			SoC	2	2 (100.0)	1657.09 (159.93)	1544.0	1657.09	1770.2	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeFF, created on: 13SEP2023

Table PT1LDHWC_SIHB: Change from baseline in LDH (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LDH level (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	2058.17 (834.26)	876.0	1915.50	4305.0	
			SoC	16	16 (100.0)	1974.59 (1060.41)	630.0	2033.25	4895.5	
	Week 2	Peg	23	22 (95.7)	251.45 (125.14)	129.0	223.50	753.0		
		SoC	16	15 (93.8)	1851.87 (1084.77)	438.0	1828.00	5008.0		
	Week 4	Peg	23	22 (95.7)	142.46 (22.28)	96.0	149.50	181.4		
		SoC	16	15 (93.8)	1995.73 (1202.24)	240.0	2012.00	4612.0		
	Week 6	Peg	23	22 (95.7)	149.07 (31.63)	102.0	150.50	251.5		
		SoC	16	16 (100.0)	1945.26 (1379.48)	606.0	1628.00	5582.0		
	Week 8	Peg	23	22 (95.7)	151.84 (36.70)	56.3	154.50	221.0		
		SoC	16	16 (100.0)	2036.07 (1443.57)	548.0	1831.00	5891.0		
	Week 10	Peg	23	23 (100.0)	163.60 (37.82)	58.2	167.00	219.0		
		SoC	16	16 (100.0)	1969.19 (1282.20)	664.0	1771.00	4899.0		
	Week 12	Peg	23	23 (100.0)	178.41 (31.93)	127.0	170.00	263.4		
		SoC	16	16 (100.0)	1938.34 (1215.45)	732.0	1771.00	4824.0		
	Week 14	Peg	23	23 (100.0)	177.76 (30.97)	123.0	179.00	251.5		
		SoC	16	16 (100.0)	1966.84 (1329.95)	592.0	1681.50	4964.5		
	Week 16	Peg	23	23 (100.0)	194.19 (80.73)	121.0	184.00	540.0		
		SoC	16	16 (100.0)	1927.75 (1356.45)	613.0	1661.00	5208.0		
	Week 18	Peg	23	23 (100.0)	185.76 (53.22)	122.0	179.54	368.0		
		SoC	16	16 (100.0)	2031.31 (1397.48)	719.0	1749.00	5544.0		
	Week 20	Peg	23	23 (100.0)	173.30 (37.01)	112.0	165.00	228.0		
		SoC	16	15 (93.8)	2167.71 (2110.65)	626.0	1615.00	8799.7		
	Week 22	Peg	23	22 (95.7)	179.89 (44.74)	124.0	170.00	313.0		
		SoC	16	15 (93.8)	2208.31 (2112.42)	789.0	1707.00	8799.7		
	Week 24	Peg	23	23 (100.0)	179.56 (37.40)	114.0	176.00	242.0		
		SoC	16	16 (100.0)	2101.10 (2065.29)	417.8	1727.00	8799.7		
	Week 26	Peg	23	20 (87.0)	199.79 (104.25)	109.0	179.50	612.0		
		SoC	16	12 (75.0)	2374.47 (2308.89)	626.0	1865.00	8799.7		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHB: Change from baseline in LDH (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in LDH (censored)	Latin America	Week 2	Peg	12	10 (83.3)	-2104.37 (1145.07)	-4689.0	-2026.87	-347.0	-2.14 (1.05) [-4.72, -0.61]
			SoC	2	2 (100.0)	436.92 (478.59)	98.5	436.92	775.3	
		Week 4	Peg	12	11 (91.7)	-2176.00 (1108.38)	-4808.0	-2246.09	-374.0	-2.03 (1.00) [-4.44, -0.52]
			SoC	2	2 (100.0)	139.50 (329.52)	-93.5	139.50	372.5	
		Week 6	Peg	12	11 (91.7)	-2178.58 (1111.28)	-4821.0	-2236.64	-373.0	-1.86 (0.98) [-4.19, -0.35]
			SoC	2	2 (100.0)	-59.66 (216.60)	-212.8	-59.66	93.5	
		Week 8	Peg	12	11 (91.7)	-2150.71 (1119.30)	-4800.0	-2219.62	-312.0	-1.82 (0.97) [-4.13, -0.31]
			SoC	2	2 (100.0)	-59.66 (216.60)	-212.8	-59.66	93.5	
		Week 10	Peg	12	11 (91.7)	-2147.97 (1114.96)	-4789.0	-2171.52	-291.2	-1.82 (0.97) [-4.14, -0.32]
			SoC	2	2 (100.0)	-59.66 (216.60)	-212.8	-59.66	93.5	
		Week 12	Peg	12	11 (91.7)	-2142.98 (1098.77)	-4779.0	-2190.30	-377.0	-1.85 (0.97) [-4.17, -0.34]
			SoC	2	2 (100.0)	-59.66 (216.60)	-212.8	-59.66	93.5	
		Week 14	Peg	12	11 (91.7)	-2139.99 (1120.14)	-4787.0	-2211.33	-381.0	-1.81 (0.97) [-4.12, -0.30]
			SoC	2	2 (100.0)	-59.66 (216.60)	-212.8	-59.66	93.5	
		Week 16	Peg	12	11 (91.7)	-2158.46 (1102.16)	-4782.0	-2190.33	-401.4	-1.85 (0.98) [-4.19, -0.35]
			SoC	2	2 (100.0)	-59.66 (216.60)	-212.8	-59.66	93.5	
		Week 18	Peg	12	10 (83.3)	-2092.05 (1159.94)	-4781.0	-1890.46	-393.0	-1.70 (0.99) [-4.06, -0.19]
			SoC	2	2 (100.0)	-59.66 (216.60)	-212.8	-59.66	93.5	
		Week 20	Peg	12	11 (91.7)	-2142.80 (1094.48)	-4769.0	-1931.21	-413.0	-1.85 (0.98) [-4.18, -0.35]
			SoC	2	2 (100.0)	-59.66 (216.60)	-212.8	-59.66	93.5	
Week 22	Peg	12	11 (91.7)	-2139.06 (1133.10)	-4788.0	-2111.35	-195.0	-1.79 (0.97) [-4.09, -0.28]		
	SoC	2	2 (100.0)	-59.66 (216.60)	-212.8	-59.66	93.5			

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHB: Change from baseline in LDH (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in LDH (censored)	Latin America	Week 24	Peg	12	11 (91.7)	-2127.48 (1091.30)	-4758.0	-1909.50	-434.0	-1.85 (0.97) [-4.17, -0.34]
			SoC	2	2 (100.0)	-59.66 (216.60)	-212.8	-59.66	93.5	
		Week 26	Peg	12	10 (83.3)	-2308.38 (985.93)	-4770.0	-2115.59	-1013.5	-2.21 (1.06) [-4.83, -0.68]
			SoC	2	2 (100.0)	-59.66 (216.60)	-212.8	-59.66	93.5	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHB: Change from baseline in LDH (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline in LDH (censored)	Asia Pacific	Week 2	Peg	23	22 (95.7)	-1800.20 (858.75)	-3967.0	-1622.00	-636.0	-2.25 (0.45) [-3.26, -1.51]	
			SoC	16	15 (93.8)	-188.93 (350.62)	-1000.0	-61.00	127.5		
			Week 4	Peg	23	22 (95.7)	-1945.24 (840.25)	-4155.0	-1827.25	-719.0	-2.48 (0.47) [-3.55, -1.72]
		SoC		16	15 (93.8)	-45.07 (584.20)	-1043.0	-135.50	1282.0		
			Week 6	Peg	23	22 (95.7)	-1879.74 (838.71)	-4151.0	-1715.00	-724.0	-2.17 (0.43) [-3.15, -1.45]
		SoC		16	16 (100.0)	-29.33 (830.20)	-1194.0	-40.40	2399.5		
			Week 8	Peg	23	22 (95.7)	-1899.82 (854.12)	-4147.0	-1704.00	-673.0	-2.28 (0.44) [-3.28, -1.55]
		SoC		16	16 (100.0)	61.47 (825.01)	-1043.0	-85.25	2399.5		
			Week 10	Peg	23	23 (100.0)	-1894.57 (829.31)	-4125.0	-1768.50	-684.0	-2.28 (0.44) [-3.26, -1.56]
		SoC		16	16 (100.0)	-5.40 (788.57)	-1043.0	-85.25	2399.5		
			Week 12	Peg	23	23 (100.0)	-1879.77 (841.68)	-4144.0	-1742.50	-668.0	-2.20 (0.43) [-3.17, -1.48]
		SoC		16	16 (100.0)	-36.26 (791.43)	-1043.0	-85.25	2399.5		
			Week 14	Peg	23	23 (100.0)	-1880.42 (838.08)	-4122.0	-1718.50	-682.0	-2.23 (0.43) [-3.20, -1.51]
		SoC		16	16 (100.0)	-7.75 (802.69)	-1043.0	-85.25	2399.5		
			Week 16	Peg	23	23 (100.0)	-1863.98 (845.54)	-4131.0	-1595.50	-662.0	-2.16 (0.43) [-3.13, -1.45]
		SoC		16	16 (100.0)	-46.84 (787.72)	-1043.0	-50.75	2399.5		
			Week 18	Peg	23	23 (100.0)	-1872.41 (849.81)	-4139.0	-1697.50	-622.0	-2.27 (0.44) [-3.25, -1.55]
		SoC		16	16 (100.0)	56.72 (809.12)	-1043.0	-71.50	2399.5		
			Week 20	Peg	23	23 (100.0)	-1884.87 (842.36)	-4156.0	-1752.50	-650.0	-1.85 (0.41) [-2.77, -1.15]
		SoC		16	15 (93.8)	126.91 (1345.67)	-1091.0	-84.50	3904.2		
		Week 22	Peg	23	22 (95.7)	-1871.77 (859.82)	-4123.0	-1697.50	-664.0	-1.85 (0.42) [-2.78, -1.15]	
	SoC		16	15 (93.8)	167.51 (1344.82)	-1194.0	-84.50	3904.2			

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHB: Change from baseline in LDH (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in LDH (censored)	Asia	Week 24	Peg	23	23 (100.0)	-1878.61 (838.91)	-4101.0	-1778.50	-660.0	-1.84 (0.40) [-2.74, -1.16]
			SoC	16	16 (100.0)	126.50 (1334.13)	-1614.7	-85.25	3904.2	
	Pacific	Week 26	Peg	23	20 (87.0)	-1931.46 (892.13)	-4100.0	-1975.75	-675.0	-2.07 (0.47) [-3.16, -1.30]
			SoC	16	12 (75.0)	392.14 (1370.47)	-1026.0	-44.25	3904.2	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SICA: Change from baseline in LDH (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.1908

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 LDH = Lactate dehydrogenase levels (U/l).
 Source Data: aeфф, created on: 13SEP2023

Table PT1LDHWC_SICA: Change from baseline in LDH (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 2	21	18 (85.7)	-1933.45 (88.24)	(-2116.45, -1750.44)	-1719.54 (167.15)	(-2066.19, -1372.89)	<0.0001	*
		Week 4	21	19 (90.5)	-2051.42 (65.07)	(-2186.03, -1916.81)	-1662.69 (126.00)	(-1923.35, -1402.03)	<0.0001	*
		Week 6	21	20 (95.2)	-1978.05 (52.34)	(-2085.84, -1870.27)	-1379.71 (98.85)	(-1583.29, -1176.13)	<0.0001	*
		Week 8	21	19 (90.5)	-1965.86 (64.81)	(-2099.63, -1832.09)	-1451.48 (120.17)	(-1699.51, -1203.46)	<0.0001	*
		Week 10	21	20 (95.2)	-1960.95 (59.10)	(-2082.68, -1839.22)	-1436.00 (111.63)	(-1665.90, -1206.09)	<0.0001	*
		Week 12	21	20 (95.2)	-1944.12 (54.03)	(-2055.39, -1832.84)	-1422.87 (102.05)	(-1633.03, -1212.70)	<0.0001	*
		Week 14	21	20 (95.2)	-1954.39 (73.51)	(-2105.78, -1803.00)	-1414.20 (138.83)	(-1700.13, -1128.28)	<0.0001	*
		Week 16	21	20 (95.2)	-1933.47 (81.74)	(-2101.82, -1765.12)	-1383.33 (154.38)	(-1701.29, -1065.37)	<0.0001	*
		Week 18	21	20 (95.2)	-1920.53 (69.36)	(-2063.37, -1777.69)	-1434.20 (130.99)	(-1703.98, -1164.42)	<0.0001	*
		Week 20	21	20 (95.2)	-1981.16 (75.70)	(-2137.39, -1824.93)	-1404.90 (149.11)	(-1712.65, -1097.14)	<0.0001	*
		Week 22	21	19 (90.5)	-1984.97 (91.12)	(-2173.46, -1796.48)	-1454.06 (176.12)	(-1818.39, -1089.73)	<0.0001	*
		Week 24	21	20 (95.2)	-1945.31 (70.34)	(-2090.17, -1800.45)	-1235.75 (132.84)	(-1509.35, -962.15)	<0.0001	*
		Week 26	21	18 (85.7)	-1972.53 (71.34)	(-2121.34, -1823.72)	-1402.77 (157.19)	(-1730.67, -1074.88)	<0.0001	*
		SoC	Week 2	8	7 (87.5)	-213.91 (141.70)	(-507.78, 79.97)			
	Week 4		8	7 (87.5)	-388.73 (107.53)	(-611.17, -166.29)				
	Week 6		8	8 (100.0)	-598.35 (83.23)	(-769.75, -426.94)				
	Week 8		8	8 (100.0)	-514.37 (100.42)	(-721.64, -307.11)				
	Week 10		8	8 (100.0)	-524.96 (93.99)	(-718.53, -331.38)				
	Week 12		8	8 (100.0)	-521.25 (85.92)	(-698.20, -344.30)				
	Week 14		8	8 (100.0)	-540.18 (116.89)	(-780.92, -299.44)				
	Week 16		8	8 (100.0)	-550.14 (129.99)	(-817.85, -282.43)				
	Week 18		8	8 (100.0)	-486.33 (110.29)	(-713.48, -259.18)				
	Week 20		8	7 (87.5)	-576.27 (128.20)	(-840.86, -311.67)				
	Week 22		8	7 (87.5)	-530.91 (150.39)	(-842.03, -219.80)				
	Week 24		8	8 (100.0)	-709.56 (111.85)	(-939.92, -479.20)				
	Week 26	8	5 (62.5)	-569.75 (138.04)	(-857.70, -281.80)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 LDH = Lactate dehydrogenase levels (U/l).
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SICA: Change from baseline in LDH (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 2	14	14 (100.0)	-1779.32 (161.59)	(-2115.36, -1443.28)	-1639.46 (250.48)	(-2160.37, -1118.55)	<0.0001	*
		Week 4	14	14 (100.0)	-1895.67 (189.41)	(-2289.57, -1501.76)	-2006.57 (293.62)	(-2617.19, -1395.95)	<0.0001	*
		Week 6	14	13 (92.9)	-1854.92 (242.38)	(-2360.50, -1349.33)	-2110.36 (367.64)	(-2877.24, -1343.48)	<0.0001	*
		Week 8	14	14 (100.0)	-1888.06 (237.73)	(-2382.46, -1393.67)	-2219.01 (368.52)	(-2985.40, -1452.62)	<0.0001	*
		Week 10	14	14 (100.0)	-1874.15 (216.98)	(-2325.37, -1422.92)	-2098.97 (336.35)	(-2798.44, -1399.50)	<0.0001	*
		Week 12	14	14 (100.0)	-1866.53 (210.95)	(-2305.23, -1427.83)	-2034.20 (327.01)	(-2714.26, -1354.15)	<0.0001	*
		Week 14	14	14 (100.0)	-1857.35 (221.11)	(-2317.18, -1397.52)	-2095.27 (342.76)	(-2808.08, -1382.46)	<0.0001	*
		Week 16	14	14 (100.0)	-1875.54 (220.91)	(-2334.94, -1416.13)	-2059.99 (342.44)	(-2772.14, -1347.84)	<0.0001	*
		Week 18	14	13 (92.9)	-1842.68 (247.44)	(-2358.83, -1326.52)	-2148.51 (375.39)	(-2931.56, -1365.46)	<0.0001	*
		Week 20	14	14 (100.0)	-1893.57 (336.11)	(-2592.55, -1194.60)	-2396.44 (521.02)	(-3479.96, -1312.93)	0.0002	*
		Week 22	14	14 (100.0)	-1871.96 (335.41)	(-2569.48, -1174.45)	-2404.28 (519.93)	(-3485.54, -1323.02)	0.0001	*
		Week 24	14	14 (100.0)	-1868.64 (327.23)	(-2549.16, -1188.13)	-2472.44 (507.26)	(-3527.35, -1417.53)	<0.0001	*
		Week 26	14	12 (85.7)	-2029.76 (368.97)	(-2804.93, -1254.59)	-2650.30 (564.87)	(-3837.05, -1463.55)	0.0002	*
		SoC	Week 2	10	10 (100.0)	-139.85 (191.23)	(-537.53, 257.82)			
	Week 4		10	10 (100.0)	110.91 (224.16)	(-355.25, 577.07)				
	Week 6		10	10 (100.0)	255.44 (276.36)	(-321.04, 831.92)				
	Week 8		10	10 (100.0)	330.95 (281.34)	(-254.13, 916.03)				
	Week 10		10	10 (100.0)	224.83 (256.78)	(-309.17, 758.82)				
	Week 12		10	10 (100.0)	167.68 (249.65)	(-351.49, 686.85)				
	Week 14		10	10 (100.0)	237.92 (261.67)	(-306.26, 782.10)				
	Week 16		10	10 (100.0)	184.46 (261.43)	(-359.22, 728.13)				
	Week 18		10	10 (100.0)	305.83 (282.15)	(-282.72, 894.38)				
	Week 20		10	10 (100.0)	502.87 (397.76)	(-324.31, 1330.05)				
	Week 22		10	10 (100.0)	532.32 (396.93)	(-293.14, 1357.78)				
	Week 24		10	10 (100.0)	603.80 (387.26)	(-201.54, 1409.14)				
	Week 26	10	9 (90.0)	620.54 (426.29)	(-275.06, 1516.13)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 LDH = Lactate dehydrogenase levels (U/l).
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHA: Change from baseline in LDH (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LDH level (censored) < 4	Baseline	Peg	21	21 (100.0)	2197.62 (863.60)	1121.5	2061.29	4946.0	
		SoC	8	8 (100.0)	1899.38 (752.86)	630.0	2033.25	2835.0	
	Week 2	Peg	21	18 (85.7)	211.83 (39.53)	157.0	206.50	283.0	
		SoC	8	7 (87.5)	1909.62 (755.15)	438.0	2027.00	2758.3	
	Week 4	Peg	21	19 (90.5)	138.34 (21.89)	96.0	141.84	181.4	
		SoC	8	7 (87.5)	1778.22 (583.11)	591.0	1994.00	2355.5	
	Week 6	Peg	21	20 (95.2)	143.79 (32.95)	102.0	134.50	251.5	
		SoC	8	8 (100.0)	1492.55 (469.15)	606.0	1628.00	1952.0	
	Week 8	Peg	21	19 (90.5)	155.90 (29.29)	116.0	151.00	221.0	
		SoC	8	8 (100.0)	1566.78 (564.86)	548.0	1832.59	1999.0	
	Week 10	Peg	21	20 (95.2)	164.06 (27.49)	111.0	162.00	211.0	
		SoC	8	8 (100.0)	1558.03 (546.65)	664.0	1692.59	2197.0	
	Week 12	Peg	21	20 (95.2)	179.67 (31.99)	129.0	170.00	274.0	
		SoC	8	8 (100.0)	1564.80 (497.07)	732.0	1692.59	2187.0	
	Week 14	Peg	21	20 (95.2)	172.94 (29.01)	123.0	171.50	232.1	
		SoC	8	8 (100.0)	1537.02 (675.62)	592.0	1635.50	2700.0	
	Week 16	Peg	21	20 (95.2)	193.57 (85.99)	121.0	181.50	540.0	
		SoC	8	8 (100.0)	1527.77 (725.47)	613.0	1493.00	2913.0	
	Week 18	Peg	21	20 (95.2)	205.68 (122.29)	125.0	180.27	700.0	
		SoC	8	8 (100.0)	1593.65 (604.02)	719.0	1692.59	2631.0	
	Week 20	Peg	21	20 (95.2)	179.52 (69.29)	112.0	164.00	441.1	
		SoC	8	7 (87.5)	1562.17 (703.30)	626.0	1615.00	2755.0	
	Week 22	Peg	21	19 (90.5)	174.01 (38.00)	124.0	168.00	261.0	
		SoC	8	7 (87.5)	1606.31 (805.76)	811.0	1615.00	3123.0	
	Week 24	Peg	21	20 (95.2)	177.32 (35.79)	114.0	179.22	241.1	
		SoC	8	8 (100.0)	1379.38 (617.60)	417.8	1681.00	1967.0	
	Week 26	Peg	21	18 (85.7)	180.84 (43.52)	109.0	171.34	270.0	
		SoC	8	5 (62.5)	1501.24 (731.57)	626.0	1770.18	2314.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHA: Change from baseline in LDH (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LDH level (censored) >= 4	Baseline	Peg	14	14 (100.0)	2080.93 (1003.41)	538.0	2262.50	4305.0	
		SoC	10	10 (100.0)	1983.20 (1207.77)	826.0	1725.25	4895.5	
	Week 2	Peg	14	14 (100.0)	281.29 (153.93)	129.0	245.50	753.0	
		SoC	10	10 (100.0)	1871.80 (1244.49)	674.0	1641.00	5008.0	
	Week 4	Peg	14	14 (100.0)	166.70 (63.11)	91.0	155.00	337.0	
		SoC	10	10 (100.0)	2120.10 (1423.87)	240.0	2175.50	4612.0	
	Week 6	Peg	14	13 (92.9)	169.50 (58.58)	114.0	154.00	330.0	
		SoC	10	10 (100.0)	2249.80 (1654.24)	824.0	1628.00	5582.0	
	Week 8	Peg	14	14 (100.0)	177.48 (71.20)	56.3	163.50	351.0	
		SoC	10	10 (100.0)	2335.70 (1721.70)	789.0	1737.00	5891.0	
	Week 10	Peg	14	14 (100.0)	187.01 (58.37)	58.2	186.00	309.0	
		SoC	10	10 (100.0)	2235.70 (1517.54)	789.0	1771.00	4899.0	
	Week 12	Peg	14	14 (100.0)	192.95 (49.24)	127.0	174.36	311.0	
		SoC	10	10 (100.0)	2180.91 (1449.49)	789.0	1771.00	4824.0	
	Week 14	Peg	14	14 (100.0)	203.85 (58.42)	135.0	185.50	351.0	
		SoC	10	10 (100.0)	2248.75 (1537.72)	789.0	1771.00	4964.5	
	Week 16	Peg	14	14 (100.0)	186.86 (37.82)	129.0	185.00	274.0	
		SoC	10	10 (100.0)	2193.60 (1567.04)	789.0	1771.00	5208.0	
	Week 18	Peg	14	13 (92.9)	201.69 (68.78)	122.0	172.00	368.0	
		SoC	10	10 (100.0)	2306.60 (1658.51)	789.0	1771.00	5544.0	
	Week 20	Peg	14	14 (100.0)	184.93 (42.28)	125.0	173.00	260.0	
		SoC	10	10 (100.0)	2489.47 (2502.11)	789.0	1625.50	8799.7	
	Week 22	Peg	14	14 (100.0)	206.14 (61.33)	130.0	179.00	343.0	
		SoC	10	10 (100.0)	2519.47 (2487.86)	789.0	1758.50	8799.7	
	Week 24	Peg	14	14 (100.0)	210.38 (85.65)	104.0	202.00	473.3	
		SoC	10	10 (100.0)	2589.67 (2476.05)	789.0	1771.00	8799.7	
	Week 26	Peg	14	12 (85.7)	240.25 (126.97)	131.0	203.00	612.0	
		SoC	10	9 (90.0)	2700.19 (2568.74)	789.0	1835.00	8799.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHA: Change from baseline in LDH (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in LDH (censored)	Week 2	Peg	21	18 (85.7)	-1969.62 (923.46)	-4689.0	-1789.00	-878.5	-2.13 (0.58) [-3.48, -1.21]
		SoC	8	7 (87.5)	-120.88 (522.57)	-990.0	-192.00	775.3	
	Week 4	Peg	21	19 (90.5)	-2101.69 (888.94)	-4808.0	-1919.45	-969.5	-2.22 (0.58) [-3.57, -1.30]
		SoC	8	7 (87.5)	-252.28 (470.57)	-1043.0	-39.00	372.5	
	Week 6	Peg	21	20 (95.2)	-2054.66 (889.13)	-4821.0	-1906.87	-993.5	-2.01 (0.53) [-3.22, -1.14]
		SoC	8	8 (100.0)	-406.83 (465.25)	-1194.0	-194.66	-24.0	
	Week 8	Peg	21	19 (90.5)	-2042.40 (912.43)	-4800.0	-1869.33	-978.5	-2.07 (0.54) [-3.32, -1.19]
		SoC	8	8 (100.0)	-332.59 (381.29)	-1043.0	-153.70	-82.0	
	Week 10	Peg	21	20 (95.2)	-2034.40 (882.42)	-4789.0	-1892.87	-969.5	-2.12 (0.54) [-3.35, -1.24]
		SoC	8	8 (100.0)	-341.34 (356.25)	-1043.0	-194.13	34.0	
	Week 12	Peg	21	20 (95.2)	-2018.79 (883.41)	-4779.0	-1875.17	-958.5	-2.09 (0.54) [-3.32, -1.22]
		SoC	8	8 (100.0)	-334.57 (385.40)	-1043.0	-204.41	102.0	
	Week 14	Peg	21	20 (95.2)	-2025.52 (884.41)	-4787.0	-1889.73	-953.5	-2.07 (0.53) [-3.30, -1.20]
		SoC	8	8 (100.0)	-362.35 (359.38)	-1043.0	-223.66	-38.0	
	Week 16	Peg	21	20 (95.2)	-2004.89 (896.91)	-4782.0	-1860.12	-937.5	-1.99 (0.53) [-3.20, -1.13]
		SoC	8	8 (100.0)	-371.60 (417.54)	-1043.0	-202.16	78.0	
	Week 18	Peg	21	20 (95.2)	-1992.78 (885.71)	-4781.0	-1841.00	-939.5	-2.10 (0.54) [-3.33, -1.22]
		SoC	8	8 (100.0)	-305.73 (373.68)	-1043.0	-208.41	89.0	
	Week 20	Peg	21	20 (95.2)	-2018.94 (884.09)	-4769.0	-1895.04	-956.5	-1.87 (0.54) [-3.09, -0.97]
		SoC	8	7 (87.5)	-468.33 (479.89)	-1091.0	-212.82	-4.0	
Week 22	Peg	21	19 (90.5)	-2024.29 (911.62)	-4788.0	-1880.00	-953.5	-1.83 (0.54) [-3.07, -0.93]	
	SoC	8	7 (87.5)	-424.19 (610.58)	-1194.0	-212.82	288.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHA: Change from baseline in LDH (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in LDH (censored)	Week 24	Peg	21	20 (95.2)	-2021.13 (884.95)	-4758.0	-1866.14	-956.5	-1.79 (0.51) [-2.94, -0.94]
		SoC	8	8 (100.0)	-520.00 (590.74)	-1614.7	-249.91	79.0	
	Week 26	Peg	21	18 (85.7)	-2077.22 (915.75)	-4770.0	-1975.75	-948.5	-2.19 (0.65) [-3.70, -1.15]
		SoC	8	5 (62.5)	-192.86 (198.69)	-521.0	-140.50	-4.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHA: Change from baseline in LDH (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in LDH (censored)	Week 2	Peg	14	14 (100.0)	-1799.64 (1007.60)	-3967.0	-2001.50	-347.0	-2.03 (0.55) [-3.31, -1.16]
		SoC	10	10 (100.0)	-111.40 (333.76)	-1000.0	-27.00	127.5	
	Week 4	Peg	14	14 (100.0)	-1914.23 (999.80)	-4155.0	-2082.50	-374.0	-2.32 (0.58) [-3.69, -1.42]
		SoC	10	10 (100.0)	136.90 (576.82)	-633.5	-114.50	1282.0	
	Week 6	Peg	14	13 (92.9)	-1863.50 (1019.32)	-4151.0	-1892.02	-373.0	-2.14 (0.57) [-3.49, -1.25]
		SoC	10	10 (100.0)	266.60 (872.81)	-1026.0	133.25	2399.5	
	Week 8	Peg	14	14 (100.0)	-1903.45 (1006.46)	-4147.0	-2069.60	-312.0	-2.26 (0.57) [-3.62, -1.37]
		SoC	10	10 (100.0)	352.50 (893.13)	-1026.0	164.75	2399.5	
	Week 10	Peg	14	14 (100.0)	-1893.91 (1003.18)	-4125.0	-2045.72	-291.2	-2.17 (0.56) [-3.50, -1.29]
		SoC	10	10 (100.0)	252.50 (877.12)	-1026.0	88.25	2399.5	
	Week 12	Peg	14	14 (100.0)	-1887.98 (1007.85)	-4144.0	-2053.05	-377.0	-2.09 (0.56) [-3.39, -1.22]
		SoC	10	10 (100.0)	197.71 (890.96)	-1026.0	60.75	2399.5	
	Week 14	Peg	14	14 (100.0)	-1877.08 (1017.46)	-4122.0	-2061.32	-381.0	-2.14 (0.56) [-3.46, -1.26]
		SoC	10	10 (100.0)	265.55 (885.69)	-1026.0	85.25	2399.5	
	Week 16	Peg	14	14 (100.0)	-1894.07 (1005.22)	-4131.0	-2073.28	-401.4	-2.14 (0.56) [-3.46, -1.26]
		SoC	10	10 (100.0)	210.40 (858.23)	-1026.0	88.25	2399.5	
	Week 18	Peg	14	13 (92.9)	-1856.19 (1052.02)	-4139.0	-1904.50	-393.0	-2.13 (0.57) [-3.48, -1.24]
		SoC	10	10 (100.0)	323.40 (891.61)	-1026.0	88.25	2399.5	
	Week 20	Peg	14	14 (100.0)	-1896.00 (1004.20)	-4156.0	-2072.50	-413.0	-1.89 (0.53) [-3.13, -1.03]
		SoC	10	10 (100.0)	506.27 (1496.92)	-1026.0	88.25	3904.2	
	Week 22	Peg	14	14 (100.0)	-1874.79 (1028.40)	-4123.0	-2055.50	-195.0	-1.90 (0.54) [-3.14, -1.04]
		SoC	10	10 (100.0)	536.27 (1469.02)	-1026.0	88.25	3904.2	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1LDHWC_SIHA: Change from baseline in LDH (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in LDH (censored)	Week 24	Peg	14	14 (100.0)	-1870.55 (991.77)	-4101.0	-1908.36	-434.0	-2.00 (0.55) [-3.27, -1.13]
		SoC	10	10 (100.0)	606.47 (1442.40)	-1026.0	104.50	3904.2	
	Week 26	Peg	14	12 (85.7)	-2026.92 (979.02)	-4100.0	-2217.25	-675.0	-2.04 (0.59) [-3.45, -1.12]
		SoC	10	9 (90.0)	616.74 (1532.65)	-1026.0	83.00	3904.2	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 LDH = Lactate dehydrogenase levels (U/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1RCWC_SICC: Change from baseline in ARC (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.6134

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 ARC = Absolute reticulocyte counts (10**⁻⁹/l).
 Source Data: aeфф, created on: 13SEP2023

Table PT1RCWC_SICC: Change from baseline in ARC (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 60	Peg	Week 2	29	27 (93.1)	-128.31 (10.67)	(-150.01, -106.62)	-109.93 (20.10)	(-150.77, -69.09)	<0.0001	*
		Week 4	29	24 (82.8)	-142.46 (11.81)	(-166.57, -118.34)	-128.19 (21.97)	(-173.07, -83.31)	<0.0001	*
		Week 6	29	22 (75.9)	-136.93 (13.49)	(-164.57, -109.29)	-116.78 (24.29)	(-166.53, -67.03)	<0.0001	*
		Week 8	29	18 (62.1)	-136.87 (14.51)	(-166.87, -106.86)	-144.22 (25.71)	(-197.40, -91.03)	<0.0001	*
		Week 10	29	17 (58.6)	-131.41 (13.03)	(-158.30, -104.52)	-118.68 (21.20)	(-162.44, -74.92)	<0.0001	*
		Week 12	29	18 (62.1)	-120.60 (10.93)	(-143.17, -98.04)	-98.12 (18.48)	(-136.26, -59.98)	<0.0001	*
		Week 14	29	18 (62.1)	-113.16 (13.20)	(-140.39, -85.92)	-109.92 (22.19)	(-155.72, -64.13)	<0.0001	*
		Week 16	29	20 (69.0)	-115.84 (12.21)	(-140.98, -90.71)	-118.33 (21.70)	(-163.01, -73.64)	<0.0001	*
		Week 18	29	23 (79.3)	-115.69 (11.13)	(-138.41, -92.97)	-114.84 (19.53)	(-154.72, -74.96)	<0.0001	*
		Week 20	29	22 (75.9)	-127.27 (9.05)	(-145.81, -108.73)	-90.45 (16.20)	(-123.63, -57.26)	<0.0001	*
		Week 22	29	24 (82.8)	-126.43 (8.45)	(-143.69, -109.17)	-98.59 (15.58)	(-130.40, -66.77)	<0.0001	*
		Week 24	29	25 (86.2)	-119.36 (8.45)	(-136.62, -102.09)	-89.47 (16.48)	(-123.12, -55.82)	<0.0001	*
		Week 26	29	21 (72.4)	-125.24 (10.07)	(-145.98, -104.49)	-102.05 (19.45)	(-142.11, -61.99)	<0.0001	*
		SoC	Week 2	13	11 (84.6)	-18.38 (16.85)	(-52.63, 15.87)			
	Week 4		13	10 (76.9)	-14.27 (18.39)	(-51.83, 23.29)				
	Week 6		13	10 (76.9)	-20.14 (19.94)	(-60.99, 20.70)				
	Week 8		13	9 (69.2)	7.35 (20.62)	(-35.30, 49.99)				
	Week 10		13	11 (84.6)	-12.73 (16.05)	(-45.86, 20.40)				
	Week 12		13	10 (76.9)	-22.48 (14.57)	(-52.56, 7.59)				
	Week 14		13	10 (76.9)	-3.23 (17.52)	(-39.40, 32.93)				
	Week 16		13	9 (69.2)	2.48 (17.81)	(-34.21, 39.17)				
	Week 18		13	11 (84.6)	-0.85 (16.06)	(-33.65, 31.95)				
	Week 20		13	10 (76.9)	-36.82 (13.41)	(-64.29, -9.36)				
	Week 22		13	10 (76.9)	-27.84 (13.07)	(-54.54, -1.14)				
	Week 24		13	9 (69.2)	-29.88 (14.07)	(-58.61, -1.16)				
	Week 26	13	8 (61.5)	-23.19 (16.38)	(-56.91, 10.54)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.

ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.

ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to

Peg. NE = not evaluable. IA = p-value of interaction term.

ARC = Absolute reticulocyte counts (10⁹/l).

Source Data: aeff, created on: 13SEP2023

Table PT1RCWC_SICC: Change from baseline in ARC (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 2	6	4 (66.7)	-162.73 (40.86)	(-267.75, -57.70)	-102.66 (62.53)	(-263.39, 58.06)	0.1615	
		Week 4	6	5 (83.3)	-190.31 (19.45)	(-240.31, -140.31)	-171.00 (32.81)	(-255.35, -86.66)	0.0034	*
		Week 6	6	5 (83.3)	-191.84 (28.72)	(-262.11, -121.56)	-200.67 (46.34)	(-314.05, -87.29)	0.0049	*
		Week 8	6	5 (83.3)	-183.42 (19.67)	(-231.56, -135.29)	-141.85 (31.73)	(-219.50, -64.19)	0.0042	*
		Week 10	6	5 (83.3)	-173.68 (20.90)	(-224.83, -122.54)	-130.37 (33.72)	(-212.88, -47.86)	0.0083	*
		Week 12	6	5 (83.3)	-146.61 (17.04)	(-188.31, -104.91)	-106.22 (27.50)	(-173.50, -38.94)	0.0083	*
		Week 14	6	5 (83.3)	-181.68 (15.23)	(-218.96, -144.41)	-136.37 (25.41)	(-198.55, -74.19)	0.0017	*
		Week 16	6	5 (83.3)	-192.63 (12.59)	(-223.43, -161.83)	-148.26 (21.00)	(-199.63, -96.88)	0.0004	*
		Week 18	6	5 (83.3)	-188.31 (16.50)	(-228.70, -147.92)	-159.62 (27.53)	(-226.98, -92.26)	0.0012	*
		Week 20	6	5 (83.3)	-198.34 (15.59)	(-236.50, -160.19)	-155.69 (26.01)	(-219.33, -92.05)	0.0010	*
		Week 22	6	4 (66.7)	-199.93 (19.24)	(-249.39, -150.47)	-155.41 (29.71)	(-231.78, -79.04)	0.0034	*
		Week 24	6	5 (83.3)	-196.32 (13.31)	(-228.88, -163.76)	-169.64 (22.19)	(-223.95, -115.33)	0.0003	*
		Week 26	6	5 (83.3)	-190.66 (13.37)	(-227.79, -153.53)	-145.09 (25.94)	(-217.11, -73.07)	0.0050	*
		SoC	Week 2	5	5 (100.0)	-60.07 (34.61)	(-149.04, 28.91)			
	Week 4		5	4 (80.0)	-19.31 (22.16)	(-76.26, 37.65)				
	Week 6		5	5 (100.0)	8.84 (28.72)	(-61.44, 79.11)				
	Week 8		5	5 (100.0)	-41.58 (19.67)	(-89.71, 6.56)				
	Week 10		5	5 (100.0)	-43.32 (20.90)	(-94.46, 7.83)				
	Week 12		5	5 (100.0)	-40.39 (17.04)	(-82.09, 1.31)				
	Week 14		5	5 (100.0)	-45.32 (15.23)	(-82.59, -8.04)				
Week 16	5		5 (100.0)	-44.37 (12.59)	(-75.17, -13.57)					
Week 18	5		5 (100.0)	-28.69 (16.50)	(-69.08, 11.70)					
Week 20	5		5 (100.0)	-42.66 (15.59)	(-80.81, -4.50)					
Week 22	5	5 (100.0)	-44.52 (16.17)	(-86.09, -2.94)						
Week 24	5	5 (100.0)	-26.68 (13.31)	(-59.24, 5.88)						
Week 26	5	3 (60.0)	-45.57 (18.63)	(-97.30, 6.16)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.

ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.

ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to

Peg. NE = not evaluable. IA = p-value of interaction term.

ARC = Absolute reticulocyte counts (10**⁻⁹/l).

Source Data: aeff, created on: 13SEP2023

Table PT1RCWC_SIHC: Change from baseline in ARC (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
ARC level (censored) < 60	Baseline	Peg	29	29 (100.0)	221.26 (81.09)	45.0	235.00	403.3	
		SoC	13	13 (100.0)	192.69 (119.42)	35.0	185.00	510.0	
	Week 2	Peg	29	27 (93.1)	87.78 (30.55)	20.0	80.00	150.0	
		SoC	13	11 (84.6)	195.45 (131.78)	20.0	160.00	500.0	
	Week 4	Peg	29	24 (82.8)	70.42 (29.41)	20.0	65.00	160.0	
		SoC	13	10 (76.9)	194.00 (145.92)	10.0	165.00	500.0	
	Week 6	Peg	29	22 (75.9)	72.27 (25.06)	30.0	70.00	150.0	
		SoC	13	10 (76.9)	185.00 (139.46)	20.0	155.00	530.0	
	Week 8	Peg	29	18 (62.1)	81.67 (31.48)	40.0	75.00	130.0	
		SoC	13	9 (69.2)	225.56 (155.49)	30.0	180.00	470.0	
	Week 10	Peg	29	17 (58.6)	91.18 (31.00)	20.0	90.00	130.0	
		SoC	13	11 (84.6)	203.18 (125.23)	30.0	185.00	460.0	
	Week 12	Peg	29	18 (62.1)	108.89 (29.48)	40.0	110.00	150.0	
		SoC	13	10 (76.9)	200.50 (115.29)	30.0	170.00	400.0	
	Week 14	Peg	29	18 (62.1)	114.44 (29.15)	70.0	110.00	170.0	
		SoC	13	10 (76.9)	200.50 (139.53)	30.0	177.50	500.0	
	Week 16	Peg	29	20 (69.0)	108.00 (32.05)	70.0	110.00	190.0	
		SoC	13	9 (69.2)	199.44 (152.45)	30.0	185.00	540.0	
	Week 18	Peg	29	23 (79.3)	93.48 (27.73)	40.0	90.00	160.0	
		SoC	13	11 (84.6)	196.82 (136.35)	30.0	170.00	480.0	
	Week 20	Peg	29	22 (75.9)	90.45 (26.45)	40.0	90.00	130.0	
		SoC	13	10 (76.9)	173.50 (107.03)	30.0	167.50	430.0	
	Week 22	Peg	29	24 (82.8)	87.50 (30.54)	10.0	90.00	130.0	
		SoC	13	10 (76.9)	180.50 (112.85)	30.0	165.00	430.0	
	Week 24	Peg	29	25 (86.2)	92.00 (29.86)	0.0	90.00	140.0	
		SoC	13	9 (69.2)	175.56 (118.02)	30.0	150.00	430.0	
	Week 26	Peg	29	21 (72.4)	105.24 (30.27)	60.0	100.00	190.0	
		SoC	13	8 (61.5)	184.38 (126.48)	30.0	187.50	430.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.

95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.

ARC = Absolute reticulocyte counts (10**⁻⁹/l).

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.

Source Data: aeff, created on: 13SEP2023

Table PT1RCWC_SIHC: Change from baseline in ARC (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
ARC level (censored) >= 60	Baseline	Peg	6	6 (100.0)	273.33 (71.25)	185.0	287.50	360.0	
		SoC	5	5 (100.0)	148.00 (77.18)	75.0	160.00	260.0	
	Week 2	Peg	6	4 (66.7)	85.00 (70.47)	40.0	55.00	190.0	
		SoC	5	5 (100.0)	128.00 (53.10)	70.0	160.00	170.0	
	Week 4	Peg	6	5 (83.3)	52.00 (32.71)	20.0	40.00	100.0	
		SoC	5	4 (80.0)	192.50 (46.46)	130.0	200.00	240.0	
	Week 6	Peg	6	5 (83.3)	68.00 (33.47)	30.0	70.00	120.0	
		SoC	5	5 (100.0)	174.00 (101.39)	60.0	180.00	320.0	
	Week 8	Peg	6	5 (83.3)	62.00 (32.71)	20.0	60.00	110.0	
		SoC	5	5 (100.0)	138.00 (66.11)	70.0	140.00	230.0	
	Week 10	Peg	6	5 (83.3)	72.00 (36.33)	20.0	80.00	120.0	
		SoC	5	5 (100.0)	136.00 (64.65)	70.0	150.00	230.0	
	Week 12	Peg	6	5 (83.3)	98.00 (19.24)	80.0	90.00	130.0	
		SoC	5	5 (100.0)	140.00 (66.33)	70.0	150.00	230.0	
	Week 14	Peg	6	5 (83.3)	82.00 (26.83)	40.0	80.00	110.0	
		SoC	5	5 (100.0)	130.00 (64.42)	70.0	120.00	230.0	
	Week 16	Peg	6	5 (83.3)	74.00 (28.81)	30.0	90.00	100.0	
		SoC	5	5 (100.0)	128.00 (64.96)	70.0	110.00	230.0	
	Week 18	Peg	6	5 (83.3)	76.00 (27.02)	30.0	80.00	100.0	
		SoC	5	5 (100.0)	146.00 (70.92)	70.0	150.00	230.0	
	Week 20	Peg	6	5 (83.3)	64.00 (25.10)	30.0	60.00	100.0	
		SoC	5	5 (100.0)	134.00 (64.27)	70.0	140.00	230.0	
	Week 22	Peg	6	4 (66.7)	70.00 (29.44)	30.0	75.00	100.0	
		SoC	5	5 (100.0)	128.00 (64.96)	70.0	110.00	230.0	
	Week 24	Peg	6	5 (83.3)	74.00 (36.47)	30.0	90.00	110.0	
		SoC	5	5 (100.0)	142.00 (67.60)	70.0	150.00	230.0	
	Week 26	Peg	6	5 (83.3)	84.00 (29.66)	40.0	90.00	110.0	
		SoC	5	3 (60.0)	150.00 (80.00)	70.0	150.00	230.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10**⁻⁹/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1RCWC_SIHC: Change from baseline in ARC (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in ARC (censored)	Week 2	Peg	29	27 (93.1)	-133.02 (83.80)	-333.3	-135.00	25.0	-1.64 (0.42) [-2.57, -0.91]
		SoC	13	11 (84.6)	-6.82 (47.18)	-115.0	-10.00	55.0	
	Week 4	Peg	29	24 (82.8)	-148.19 (89.27)	-303.3	-157.50	35.0	-1.79 (0.46) [-2.80, -1.01]
		SoC	13	10 (76.9)	-0.50 (53.30)	-110.0	-5.00	75.0	
	Week 6	Peg	29	22 (75.9)	-143.26 (88.79)	-303.3	-140.00	25.0	-1.56 (0.45) [-2.56, -0.79]
		SoC	13	10 (76.9)	-9.50 (69.82)	-120.0	-2.50	105.0	
	Week 8	Peg	29	18 (62.1)	-148.43 (79.77)	-273.3	-162.50	45.0	-2.13 (0.54) [-3.37, -1.27]
		SoC	13	9 (69.2)	26.67 (79.33)	-60.0	20.00	215.0	
	Week 10	Peg	29	17 (58.6)	-143.92 (76.48)	-283.3	-160.00	45.0	-2.04 (0.51) [-3.21, -1.22]
		SoC	13	11 (84.6)	2.27 (56.76)	-85.0	0.00	115.0	
	Week 12	Peg	29	18 (62.1)	-128.98 (68.62)	-263.3	-150.00	35.0	-1.74 (0.49) [-2.84, -0.93]
		SoC	13	10 (76.9)	-11.00 (60.50)	-110.0	-2.50	105.0	
	Week 14	Peg	29	18 (62.1)	-123.43 (72.09)	-303.3	-122.50	35.0	-1.90 (0.50) [-3.04, -1.08]
		SoC	13	10 (76.9)	7.50 (55.79)	-85.0	-5.00	135.0	
	Week 16	Peg	29	20 (69.0)	-129.08 (75.20)	-283.3	-125.00	45.0	-2.06 (0.52) [-3.25, -1.22]
		SoC	13	9 (69.2)	11.11 (35.60)	-35.0	0.00	75.0	
	Week 18	Peg	29	23 (79.3)	-121.59 (73.94)	-273.3	-135.00	45.0	-1.70 (0.44) [-2.69, -0.96]
		SoC	13	11 (84.6)	3.18 (65.85)	-85.0	-5.00	165.0	
	Week 20	Peg	29	22 (75.9)	-131.44 (77.84)	-283.3	-147.50	35.0	-1.41 (0.44) [-2.38, -0.65]
		SoC	13	10 (76.9)	-30.00 (47.20)	-85.0	-35.00	65.0	
Week 22	Peg	29	24 (82.8)	-129.86 (73.08)	-303.3	-150.00	45.0	-1.56 (0.44) [-2.54, -0.81]	
	SoC	13	10 (76.9)	-23.00 (46.86)	-85.0	-10.00	45.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10**⁻⁹/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeFF, created on: 13SEP2023

Table PT1RCWC_SIHC: Change from baseline in ARC (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in ARC (censored)	Week 24	Peg	29	25 (86.2)	-124.27 (74.61)	-293.3	-145.00	35.0	-1.46 (0.45) [-2.44, -0.69]
		SoC	13	9 (69.2)	-20.00 (52.38)	-85.0	-5.00	65.0	
	Week 26	Peg	29	21 (72.4)	-134.84 (75.48)	-283.3	-145.00	25.0	-1.86 (0.51) [-3.02, -1.01]
		SoC	13	8 (61.5)	-4.38 (39.95)	-80.0	-2.50	55.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10**⁻⁹/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1RCWC_SIHC: Change from baseline in ARC (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in ARC (censored)	Week 2	Peg	6	4 (66.7)	-211.25 (98.86)	-320.0	-200.00	-125.0	-2.39 (1.18) [-5.54, -1.01]
		SoC	5	5 (100.0)	-20.00 (39.21)	-90.0	-5.00	0.0	
	Week 4	Peg	6	5 (83.3)	-225.00 (82.76)	-320.0	-220.00	-115.0	-3.36 (1.46) [-7.36, -1.79]
		SoC	5	4 (80.0)	26.25 (34.49)	-20.0	35.00	55.0	
	Week 6	Peg	6	5 (83.3)	-209.00 (74.70)	-310.0	-200.00	-115.0	-3.72 (1.42) [-7.58, -2.15]
		SoC	5	5 (100.0)	26.00 (30.29)	-15.0	25.00	60.0	
	Week 8	Peg	6	5 (83.3)	-215.00 (73.31)	-300.0	-210.00	-115.0	-3.46 (1.35) [-7.10, -1.94]
		SoC	5	5 (100.0)	-10.00 (19.04)	-30.0	-5.00	10.0	
	Week 10	Peg	6	5 (83.3)	-205.00 (72.28)	-300.0	-200.00	-105.0	-3.35 (1.32) [-6.91, -1.86]
		SoC	5	5 (100.0)	-12.00 (13.51)	-30.0	-10.00	5.0	
	Week 12	Peg	6	5 (83.3)	-179.00 (71.27)	-270.0	-190.00	-105.0	-2.98 (1.22) [-6.25, -1.57]
		SoC	5	5 (100.0)	-8.00 (16.81)	-30.0	-5.00	10.0	
	Week 14	Peg	6	5 (83.3)	-209.00 (46.69)	-280.0	-210.00	-160.0	-4.87 (1.76) [-9.68, -3.00]
		SoC	5	5 (100.0)	-18.00 (18.23)	-40.0	-20.00	5.0	
	Week 16	Peg	6	5 (83.3)	-217.00 (37.68)	-270.0	-220.00	-170.0	-5.80 (2.04) [-11.42, -3.68]
		SoC	5	5 (100.0)	-20.00 (21.51)	-50.0	-20.00	5.0	
	Week 18	Peg	6	5 (83.3)	-215.00 (48.48)	-280.0	-220.00	-165.0	-4.90 (1.77) [-9.74, -3.03]
		SoC	5	5 (100.0)	-2.00 (27.06)	-30.0	-5.00	40.0	
	Week 20	Peg	6	5 (83.3)	-227.00 (50.45)	-300.0	-220.00	-170.0	-5.20 (1.86) [-10.30, -3.24]
		SoC	5	5 (100.0)	-14.00 (13.87)	-30.0	-20.00	5.0	
Week 22	Peg	6	4 (66.7)	-230.00 (49.67)	-290.0	-230.00	-170.0	-5.14 (2.04) [-10.82, -3.11]	
	SoC	5	5 (100.0)	-20.00 (21.51)	-50.0	-20.00	5.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10**9/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1RCWC_SIHC: Change from baseline in ARC (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in ARC (censored)	Week 24	Peg	6	5 (83.3)	-217.00 (35.64)	-270.0	-215.00	-170.0	-6.61 (2.29) [-12.93, -4.25]
		SoC	5	5 (100.0)	-6.00 (19.81)	-30.0	-5.00	20.0	
	Week 26	Peg	6	5 (83.3)	-207.00 (40.87)	-270.0	-210.00	-160.0	-4.80 (2.21) [-11.01, -2.76]
		SoC	5	3 (60.0)	-18.33 (12.58)	-30.0	-20.00	-5.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10**⁻⁹/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeFF, created on: 13SEP2023

Table PT1RCWC_SICD: Change from baseline in ARC (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA				
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA	
Sex	Peg	Overall									0.5329

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 ARC = Absolute reticulocyte counts (10**⁻⁹/l).
 Source Data: aeфф, created on: 13SEP2023

Table PT1RCWC_SICD: Change from baseline in ARC (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA				
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA	
Male	Peg	Week 2	19	17 (89.5)	-133.55 (8.76)	(-151.76, -115.34)	-81.89 (16.80)	(-116.82, -46.96)	<0.0001	*	
		Week 4	19	14 (73.7)	-144.80 (12.58)	(-171.22, -118.38)	-96.21 (22.81)	(-144.13, -48.29)	0.0005	*	
		Week 6	19	13 (68.4)	-143.26 (14.47)	(-173.79, -112.74)	-134.67 (25.46)	(-188.38, -80.96)	<0.0001	*	
		Week 8	19	11 (57.9)	-148.61 (11.61)	(-173.35, -123.86)	-104.56 (20.11)	(-147.43, -61.69)	0.0001	*	
		Week 10	19	10 (52.6)	-135.07 (11.71)	(-160.03, -110.11)	-90.43 (19.31)	(-131.58, -49.27)	0.0003	*	
		Week 12	19	11 (57.9)	-130.83 (8.75)	(-149.47, -112.18)	-82.46 (15.63)	(-115.76, -49.15)	<0.0001	*	
		Week 14	19	11 (57.9)	-135.90 (10.44)	(-158.04, -113.77)	-92.92 (18.77)	(-132.71, -53.12)	0.0001	*	
		Week 16	19	12 (63.2)	-141.86 (10.25)	(-163.50, -120.23)	-95.75 (18.75)	(-135.32, -56.18)	<0.0001	*	
		Week 18	19	14 (73.7)	-132.44 (9.20)	(-151.69, -113.19)	-99.25 (16.99)	(-134.81, -63.69)	<0.0001	*	
		Week 20	19	14 (73.7)	-143.75 (9.09)	(-162.84, -124.65)	-92.53 (17.19)	(-128.65, -56.41)	<0.0001	*	
		Week 22	19	14 (73.7)	-144.44 (9.11)	(-163.58, -125.29)	-89.25 (17.21)	(-125.41, -53.08)	<0.0001	*	
		Week 24	19	16 (84.2)	-134.52 (9.53)	(-154.47, -114.57)	-91.25 (19.31)	(-131.66, -50.83)	0.0001	*	
		Week 26	19	13 (68.4)	-137.76 (10.20)	(-159.38, -116.13)	-89.34 (20.05)	(-131.85, -46.83)	0.0004	*	
			SoC	Week 2	10	8 (80.0)	-51.66 (13.60)	(-79.94, -23.39)			
		Week 4		10	8 (80.0)	-48.58 (17.68)	(-85.73, -11.44)				
		Week 6		10	8 (80.0)	-8.59 (19.68)	(-50.11, 32.93)				
		Week 8		10	8 (80.0)	-44.04 (14.71)	(-75.39, -12.69)				
		Week 10		10	9 (90.0)	-44.64 (13.47)	(-73.35, -15.93)				
		Week 12		10	8 (80.0)	-48.37 (11.59)	(-73.08, -23.66)				
		Week 14		10	9 (90.0)	-42.99 (13.38)	(-71.34, -14.63)				
		Week 16		10	9 (90.0)	-46.11 (13.79)	(-75.21, -17.01)				
		Week 18		10	9 (90.0)	-33.19 (13.08)	(-60.57, -5.81)				
		Week 20		10	8 (80.0)	-51.22 (13.46)	(-79.49, -22.95)				
		Week 22		10	8 (80.0)	-55.19 (13.22)	(-82.95, -27.42)				
		Week 24		10	7 (70.0)	-43.27 (15.80)	(-76.35, -10.20)				
		Week 26	10	7 (70.0)	-48.42 (15.60)	(-81.49, -15.35)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.

ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.

ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to

Peg. NE = not evaluable. IA = p-value of interaction term.

ARC = Absolute reticulocyte counts (10^{**}-9/l).

Source Data: aeфф, created on: 13SEP2023

Table PT1RCWC_SICD: Change from baseline in ARC (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA				
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA	
Female	Peg	Week 2	16	14 (87.5)	-126.51 (15.93)	(-159.98, -93.04)	-113.34 (29.18)	(-174.65, -52.03)	0.0011	*	
		Week 4	16	15 (93.8)	-152.19 (15.60)	(-185.10, -119.27)	-173.75 (31.21)	(-239.61, -107.89)	<0.0001	*	
		Week 6	16	14 (87.5)	-145.62 (16.86)	(-181.19, -110.05)	-126.21 (32.11)	(-193.96, -58.45)	0.0011	*	
		Week 8	16	12 (75.0)	-140.91 (21.22)	(-186.41, -95.41)	-161.67 (45.08)	(-258.35, -64.98)	0.0030	*	
		Week 10	16	12 (75.0)	-139.86 (17.20)	(-176.53, -103.20)	-133.06 (35.45)	(-208.62, -57.50)	0.0019	*	
		Week 12	16	12 (75.0)	-116.80 (15.97)	(-150.84, -82.76)	-99.06 (32.91)	(-169.21, -28.91)	0.0088	*	
		Week 14	16	12 (75.0)	-113.13 (17.08)	(-149.77, -76.49)	-122.14 (36.40)	(-200.20, -44.08)	0.0047	*	
		Week 16	16	13 (81.3)	-117.89 (15.70)	(-151.56, -84.22)	-140.72 (35.51)	(-216.88, -64.57)	0.0014	*	
		Week 18	16	14 (87.5)	-117.28 (17.56)	(-154.34, -80.23)	-115.96 (33.39)	(-186.40, -45.52)	0.0029	*	
		Week 20	16	13 (81.3)	-128.91 (13.22)	(-156.94, -100.88)	-94.06 (24.57)	(-146.14, -41.98)	0.0015	*	
		Week 22	16	14 (87.5)	-121.44 (12.49)	(-147.79, -95.08)	-93.95 (23.75)	(-144.06, -43.85)	0.0010	*	
		Week 24	16	14 (87.5)	-120.67 (11.92)	(-145.81, -95.52)	-89.19 (22.65)	(-136.99, -41.40)	0.0011	*	
		Week 26	16	13 (81.3)	-129.81 (14.65)	(-161.46, -98.16)	-107.73 (34.80)	(-182.91, -32.55)	0.0085	*	
			SoC	Week 2	8	8 (100.0)	-13.17 (23.51)	(-62.55, 36.22)			
		Week 4		8	6 (75.0)	21.56 (26.03)	(-33.35, 76.48)				
		Week 6		8	7 (87.5)	-19.42 (25.49)	(-73.20, 34.37)				
		Week 8		8	6 (75.0)	20.76 (35.56)	(-55.50, 97.02)				
		Week 10		8	7 (87.5)	-6.80 (27.46)	(-65.33, 51.73)				
		Week 12		8	7 (87.5)	-17.74 (25.49)	(-72.08, 36.60)				
		Week 14		8	6 (75.0)	9.01 (28.67)	(-52.49, 70.51)				
		Week 16		8	5 (62.5)	22.84 (28.40)	(-38.08, 83.76)				
		Week 18		8	7 (87.5)	-1.32 (26.52)	(-57.28, 54.64)				
		Week 20		8	7 (87.5)	-34.85 (19.55)	(-76.29, 6.58)				
		Week 22		8	7 (87.5)	-27.48 (18.87)	(-67.29, 12.32)				
		Week 24		8	7 (87.5)	-31.48 (18.00)	(-69.45, 6.50)				
		Week 26	8	4 (50.0)	-22.08 (29.81)	(-86.48, 42.32)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.

ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.

ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to

Peg. NE = not evaluable. IA = p-value of interaction term.

ARC = Absolute reticulocyte counts (10^{**}-9/l).

Source Data: aeфф, created on: 13SEP2023

Table PT1RCWC_SIHD: Change from baseline in ARC (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
ARC level (censored)	Male	Baseline	Peg	19	19 (100.0)	238.86 (90.82)	45.0	260.00	403.3	
			SoC	10	10 (100.0)	159.00 (55.92)	85.0	165.00	260.0	
	Week 2	Peg	19	17 (89.5)	84.71 (36.59)	40.0	70.00	190.0		
		SoC	10	8 (80.0)	153.75 (39.62)	70.0	160.00	210.0		
	Week 4	Peg	19	14 (73.7)	72.14 (34.23)	30.0	65.00	160.0		
		SoC	10	8 (80.0)	155.00 (59.76)	70.0	165.00	240.0		
	Week 6	Peg	19	13 (68.4)	72.31 (20.88)	40.0	70.00	120.0		
		SoC	10	8 (80.0)	187.50 (80.67)	80.0	195.00	320.0		
	Week 8	Peg	19	11 (57.9)	75.45 (27.34)	40.0	70.00	130.0		
		SoC	10	8 (80.0)	163.75 (45.96)	80.0	160.00	230.0		
	Week 10	Peg	19	10 (52.6)	93.00 (23.59)	60.0	85.00	130.0		
		SoC	10	9 (90.0)	162.78 (44.38)	70.0	150.00	230.0		
	Week 12	Peg	19	11 (57.9)	109.09 (25.48)	80.0	110.00	150.0		
		SoC	10	8 (80.0)	165.63 (38.86)	90.0	170.00	230.0		
	Week 14	Peg	19	11 (57.9)	102.73 (23.28)	80.0	100.00	160.0		
		SoC	10	9 (90.0)	160.56 (46.40)	90.0	170.00	230.0		
	Week 16	Peg	19	12 (63.2)	96.67 (25.35)	60.0	90.00	150.0		
		SoC	10	9 (90.0)	155.00 (50.37)	90.0	150.00	230.0		
	Week 18	Peg	19	14 (73.7)	90.71 (20.93)	40.0	90.00	130.0		
		SoC	10	9 (90.0)	163.89 (54.53)	70.0	170.00	230.0		
	Week 20	Peg	19	14 (73.7)	89.29 (25.86)	40.0	95.00	130.0		
		SoC	10	8 (80.0)	156.88 (50.49)	70.0	145.00	230.0		
	Week 22	Peg	19	14 (73.7)	85.00 (28.22)	10.0	90.00	130.0		
		SoC	10	8 (80.0)	149.38 (49.31)	70.0	145.00	230.0		
	Week 24	Peg	19	16 (84.2)	91.25 (34.81)	0.0	90.00	140.0		
		SoC	10	7 (70.0)	154.29 (56.53)	70.0	150.00	230.0		
	Week 26	Peg	19	13 (68.4)	103.85 (25.34)	70.0	100.00	140.0		
		SoC	10	7 (70.0)	160.71 (57.48)	70.0	185.00	230.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10**⁻⁹/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeFF, created on: 13SEP2023

Table PT1RCWC_SIHD: Change from baseline in ARC (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
ARC level (censored) Female	Baseline		Peg	16	16 (100.0)	219.90 (68.99)	60.0	220.00	305.0	
			SoC	8	8 (100.0)	206.88 (152.99)	35.0	217.50	510.0	
	Week 2		Peg	16	14 (87.5)	90.71 (36.68)	20.0	95.00	150.0	
			SoC	8	8 (100.0)	195.00 (161.60)	20.0	150.00	500.0	
	Week 4		Peg	16	15 (93.8)	62.67 (26.31)	20.0	60.00	100.0	
			SoC	8	6 (75.0)	245.00 (170.50)	10.0	255.00	500.0	
	Week 6		Peg	16	14 (87.5)	70.71 (31.00)	30.0	70.00	150.0	
			SoC	8	7 (87.5)	174.29 (168.51)	20.0	150.00	530.0	
	Week 8		Peg	16	12 (75.0)	79.17 (37.04)	20.0	75.00	130.0	
			SoC	8	6 (75.0)	235.00 (201.67)	30.0	190.00	470.0	
	Week 10		Peg	16	12 (75.0)	81.67 (38.57)	20.0	80.00	130.0	
			SoC	8	7 (87.5)	207.14 (166.10)	30.0	150.00	460.0	
	Week 12		Peg	16	12 (75.0)	104.17 (30.29)	40.0	105.00	140.0	
			SoC	8	7 (87.5)	197.14 (150.08)	30.0	150.00	400.0	
	Week 14		Peg	16	12 (75.0)	111.67 (37.62)	40.0	110.00	170.0	
			SoC	8	6 (75.0)	201.67 (192.40)	30.0	115.00	500.0	
	Week 16		Peg	16	13 (81.3)	105.38 (40.75)	30.0	110.00	190.0	
			SoC	8	5 (62.5)	208.00 (217.88)	30.0	80.00	540.0	
	Week 18		Peg	16	14 (87.5)	90.00 (34.42)	30.0	95.00	160.0	
			SoC	8	7 (87.5)	202.86 (175.66)	30.0	150.00	480.0	
	Week 20		Peg	16	13 (81.3)	81.54 (30.23)	30.0	90.00	130.0	
			SoC	8	7 (87.5)	164.29 (133.40)	30.0	150.00	430.0	
	Week 22		Peg	16	14 (87.5)	85.00 (33.68)	30.0	80.00	130.0	
			SoC	8	7 (87.5)	178.57 (141.71)	30.0	150.00	430.0	
	Week 24		Peg	16	14 (87.5)	86.43 (27.35)	30.0	90.00	120.0	
			SoC	8	7 (87.5)	172.86 (137.20)	30.0	150.00	430.0	
	Week 26		Peg	16	13 (81.3)	98.46 (36.25)	40.0	100.00	190.0	
			SoC	8	4 (50.0)	200.00 (185.83)	30.0	170.00	430.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10**⁻⁹/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeFF, created on: 13SEP2023

Table PT1RCWC_SIHD: Change from baseline in ARC (censored) by sex
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Male in ARC (censored)	Week 2	Peg	19	17 (89.5)	-156.08 (102.43)	-333.3	-130.00	25.0	-1.59 (0.52) [-2.77, -0.74]
		SoC	10	8 (80.0)	-10.00 (42.59)	-90.0	0.00	45.0	
	Week 4	Peg	19	14 (73.7)	-169.17 (110.43)	-320.0	-170.00	35.0	-1.65 (0.55) [-2.92, -0.76]
		SoC	10	8 (80.0)	-8.75 (48.24)	-110.0	-5.00	50.0	
	Week 6	Peg	19	13 (68.4)	-166.79 (105.36)	-310.0	-200.00	25.0	-1.97 (0.59) [-3.36, -1.04]
		SoC	10	8 (80.0)	23.75 (66.96)	-120.0	42.50	105.0	
	Week 8	Peg	19	11 (57.9)	-182.58 (105.08)	-300.0	-225.00	45.0	-2.07 (0.64) [-3.59, -1.10]
		SoC	10	8 (80.0)	0.00 (38.54)	-60.0	2.50	55.0	
	Week 10	Peg	19	10 (52.6)	-176.33 (101.38)	-300.0	-197.50	45.0	-2.13 (0.64) [-3.66, -1.16]
		SoC	10	9 (90.0)	-3.33 (35.71)	-60.0	-10.00	55.0	
	Week 12	Peg	19	11 (57.9)	-161.67 (86.15)	-270.0	-175.00	35.0	-2.14 (0.65) [-3.69, -1.17]
		SoC	10	8 (80.0)	-9.38 (24.12)	-50.0	-7.50	25.0	
	Week 14	Peg	19	11 (57.9)	-174.39 (91.62)	-303.3	-185.00	35.0	-2.29 (0.64) [-3.83, -1.33]
		SoC	10	9 (90.0)	-5.56 (26.51)	-40.0	-10.00	45.0	
	Week 16	Peg	19	12 (63.2)	-179.03 (88.49)	-283.3	-192.50	45.0	-2.30 (0.62) [-3.79, -1.35]
		SoC	10	9 (90.0)	-11.11 (30.39)	-50.0	-20.00	55.0	
	Week 18	Peg	19	14 (73.7)	-155.95 (92.49)	-280.0	-175.00	45.0	-1.94 (0.56) [-3.24, -1.06]
		SoC	10	9 (90.0)	-2.22 (37.01)	-40.0	-15.00	75.0	
	Week 20	Peg	19	14 (73.7)	-166.31 (91.07)	-300.0	-185.00	35.0	-1.85 (0.57) [-3.17, -0.94]
		SoC	10	8 (80.0)	-18.13 (40.44)	-70.0	-20.00	65.0	
Week 22	Peg	19	14 (73.7)	-162.74 (91.62)	-303.3	-167.50	45.0	-1.72 (0.56) [-3.01, -0.83]	
	SoC	10	8 (80.0)	-25.63 (33.64)	-70.0	-25.00	35.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10**⁻⁹/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeFF, created on: 13SEP2023

Table PT1RCWC_SIHD: Change from baseline in ARC (censored) by sex
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Male in ARC (censored)	Week 24	Peg	19	16 (84.2)	-153.33 (88.68)	-293.3	-172.50	35.0	-1.79 (0.57) [-3.09, -0.87]
		SoC	10	7 (70.0)	-6.43 (47.06)	-80.0	-20.00	65.0	
	Week 26	Peg	19	13 (68.4)	-166.03 (85.35)	-283.3	-195.00	25.0	-2.17 (0.64) [-3.69, -1.18]
		SoC	10	7 (70.0)	-3.57 (30.24)	-30.0	-20.00	55.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10^{**}-9/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1RCWC_SIHD: Change from baseline in ARC (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in ARC (censored)	Female	Week 2	Peg	16	14 (87.5)	-127.38 (67.37)	-255.0	-137.50	0.0	-1.81 (0.56) [-3.12, -0.91]
			SoC	8	8 (100.0)	-11.88 (48.25)	-115.0	-7.50	55.0	
	Week 4	Peg	16	15 (93.8)	-154.22 (73.16)	-245.0	-175.00	30.0	-2.62 (0.70) [-4.29, -1.57]	
		SoC	8	6 (75.0)	28.33 (45.13)	-25.0	27.50	75.0		
	Week 6	Peg	16	14 (87.5)	-144.88 (72.87)	-275.0	-147.50	10.0	-1.80 (0.59) [-3.17, -0.86]	
		SoC	8	7 (87.5)	-22.14 (45.63)	-85.0	-15.00	25.0		
	Week 8	Peg	16	12 (75.0)	-144.86 (50.86)	-205.0	-162.50	-50.0	-2.53 (0.74) [-4.33, -1.45]	
		SoC	8	6 (75.0)	31.67 (91.96)	-40.0	0.00	215.0		
	Week 10	Peg	16	12 (75.0)	-142.36 (52.31)	-215.0	-160.00	-50.0	-2.41 (0.69) [-4.08, -1.39]	
		SoC	8	7 (87.5)	-0.71 (62.48)	-85.0	-5.00	115.0		
	Week 12	Peg	16	12 (75.0)	-119.86 (48.53)	-185.0	-117.50	-40.0	-1.82 (0.62) [-3.27, -0.85]	
		SoC	8	7 (87.5)	-10.71 (70.74)	-110.0	-5.00	105.0		
	Week 14	Peg	16	12 (75.0)	-112.36 (42.32)	-185.0	-110.00	-60.0	-2.12 (0.68) [-3.76, -1.09]	
		SoC	8	6 (75.0)	5.83 (71.30)	-85.0	-5.00	135.0		
	Week 16	Peg	16	13 (81.3)	-116.79 (53.02)	-190.0	-120.00	-50.0	-2.66 (0.78) [-4.56, -1.52]	
		SoC	8	5 (62.5)	20.00 (33.91)	-5.0	5.00	75.0		
	Week 18	Peg	16	14 (87.5)	-120.60 (59.18)	-195.0	-137.50	10.0	-1.87 (0.59) [-3.26, -0.93]	
		SoC	8	7 (87.5)	6.43 (76.52)	-85.0	-5.00	165.0		
	Week 20	Peg	16	13 (81.3)	-130.64 (69.66)	-265.0	-145.00	0.0	-1.54 (0.57) [-2.86, -0.61]	
		SoC	8	7 (87.5)	-32.14 (39.78)	-85.0	-5.00	5.0		
Week 22	Peg	16	14 (87.5)	-125.60 (59.38)	-185.0	-150.00	0.0	-1.85 (0.59) [-3.24, -0.91]		
	SoC	8	7 (87.5)	-17.86 (47.42)	-85.0	-5.00	45.0			

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10**9/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1RCWC_SIHD: Change from baseline in ARC (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in ARC (censored)	Female	Week 24	Peg	16	14 (87.5)	-124.17 (62.14)	-185.0	-150.00	10.0	-1.72 (0.58) [-3.06, -0.79]
			SoC	8	7 (87.5)	-23.57 (40.49)	-85.0	-5.00	5.0	
		Week 26	Peg	16	13 (81.3)	-131.41 (62.21)	-195.0	-155.00	20.0	-1.85 (0.73) [-3.56, -0.71]
			SoC	8	4 (50.0)	-16.25 (44.79)	-80.0	-5.00	25.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10^{**}-9/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1RCWC_SICB: Change from baseline in ARC (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.5686

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 ARC = Absolute reticulocyte counts (10**⁻⁹/l).
 Source Data: aeфф, created on: 13SEP2023

Table PT1RCWC_SICB: Change from baseline in ARC (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA		
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value			
Latin America	Peg	Week 2	12	9 (75.0)	-133.06 (9.25)	(-155.69, -110.42)	-31.55 (32.57)	(-111.24, 48.14)	0.3700			
		Week 4	12	9 (75.0)	-138.33 (13.03)	(-170.22, -106.44)	-12.69 (45.96)	(-125.16, 99.78)	0.7917			
		Week 6	12	7 (58.3)	-139.31 (8.54)	(-163.03, -115.59)	-79.48 (27.09)	(-154.69, -4.27)	0.0426	*		
		Week 8	12	4 (33.3)	-146.80 (12.59)	(-306.71, 13.10)	-83.26 (32.97)	(-502.24, 335.71)	0.2400			
		Week 10	12	3 (25.0)	-153.04 (40.88)	(-672.49, 366.40)	-132.49 (80.53)	(-1155.70, 890.72)	0.3477			
		Week 12	12	3 (25.0)	-130.00 (NE)	(NE, NE)	NE (NE)	(NE, NE)	NE			
		Week 14	12	3 (25.0)	-138.13 (NE)	(NE, NE)	NE (NE)	(NE, NE)	NE			
		Week 16	12	5 (41.7)	-127.35 (14.02)	(-187.68, -67.01)	-132.85 (31.02)	(-266.33, 0.63)	0.0504			
		Week 18	12	6 (50.0)	-137.96 (6.87)	(-159.84, -116.09)	-147.93 (20.61)	(-213.53, -82.33)	0.0056	*		
		Week 20	12	6 (50.0)	-177.92 (17.02)	(-232.07, -123.77)	-155.85 (50.62)	(-316.96, 5.25)	0.0542			
		Week 22	12	7 (58.3)	-153.21 (8.40)	(-176.53, -129.90)	-155.71 (26.38)	(-228.94, -82.48)	0.0041	*		
		Week 24	12	8 (66.7)	-136.25 (15.29)	(-175.55, -96.95)	NE (NE)	(NE, NE)	NE			
		Week 26	12	7 (58.3)	-142.57 (15.23)	(-184.86, -100.29)	-101.40 (45.94)	(-228.94, 26.14)	0.0919			
			SoC	Week 2	2	1 (50.0)	-101.51 (30.82)	(-176.93, -26.09)				
				Week 4	2	1 (50.0)	-125.64 (43.61)	(-232.36, -18.92)				
				Week 6	2	1 (50.0)	-59.83 (24.97)	(-129.15, 9.49)				
				Week 8	2	1 (50.0)	-63.54 (28.46)	(-425.15, 298.07)				
				Week 10	2	2 (100.0)	-20.55 (57.06)	(-745.54, 704.44)				
				Week 12	2	1 (50.0)	-35.00 (NE)	(NE, NE)				
Week 14	2			1 (50.0)	10.62 (NE)	(NE, NE)						
Week 16	2			1 (50.0)	5.50 (31.25)	(-128.97, 139.98)						
Week 18	2			1 (50.0)	9.97 (19.33)	(-51.56, 71.50)						
Week 20	2			1 (50.0)	-22.07 (44.57)	(-163.90, 119.76)						
Week 22	2			1 (50.0)	2.50 (24.34)	(-65.07, 70.07)						
Week 24	2	0 (0.0)	NE (NE)	(NE, NE)								
Week 26	2	1 (50.0)	-41.18 (43.38)	(-161.61, 79.26)								

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 ARC = Absolute reticulocyte counts (10**⁻⁹/l).
 Source Data: aeff, created on: 13SEP2023

Table PT1RCWC_SICB: Change from baseline in ARC (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Asia Pacific Peg		Week 2	23	22 (95.7)	-132.35 (12.42)	(-157.63, -107.07)	-104.64 (19.89)	(-145.10, -64.17)	<0.0001	*
		Week 4	23	20 (87.0)	-155.65 (13.56)	(-183.39, -127.91)	-150.02 (21.77)	(-194.54, -105.50)	<0.0001	*
		Week 6	23	20 (87.0)	-145.73 (14.57)	(-175.47, -115.98)	-131.39 (23.08)	(-178.52, -84.26)	<0.0001	*
		Week 8	23	19 (82.6)	-149.74 (15.51)	(-181.52, -117.96)	-144.19 (25.03)	(-195.47, -92.91)	<0.0001	*
		Week 10	23	19 (82.6)	-140.29 (12.69)	(-166.24, -114.33)	-118.93 (20.16)	(-160.16, -77.70)	<0.0001	*
		Week 12	23	20 (87.0)	-125.99 (10.52)	(-147.47, -104.51)	-99.43 (17.01)	(-134.17, -64.69)	<0.0001	*
		Week 14	23	20 (87.0)	-125.45 (12.79)	(-151.58, -99.33)	-108.39 (20.68)	(-150.63, -66.15)	<0.0001	*
		Week 16	23	20 (87.0)	-129.50 (13.29)	(-156.69, -102.32)	-113.41 (21.94)	(-158.29, -68.54)	<0.0001	*
		Week 18	23	22 (95.7)	-126.11 (12.59)	(-151.73, -100.48)	-112.89 (20.18)	(-153.94, -71.84)	<0.0001	*
		Week 20	23	21 (91.3)	-128.96 (9.37)	(-148.08, -109.85)	-86.74 (15.04)	(-117.40, -56.07)	<0.0001	*
		Week 22	23	21 (91.3)	-129.73 (10.13)	(-150.39, -109.08)	-89.75 (16.33)	(-123.06, -56.44)	<0.0001	*
		Week 24	23	22 (95.7)	-127.21 (9.68)	(-146.94, -107.48)	-92.39 (15.91)	(-124.79, -59.98)	<0.0001	*
		Week 26	23	19 (82.6)	-134.05 (11.05)	(-156.80, -111.31)	-101.56 (19.50)	(-141.73, -61.40)	<0.0001	*
		SoC		Week 2	16	15 (93.8)	-27.71 (15.08)	(-58.39, 2.96)		
Week 4	16			13 (81.3)	-5.63 (16.71)	(-39.81, 28.55)				
Week 6	16			14 (87.5)	-14.34 (17.37)	(-49.81, 21.14)				
Week 8	16			13 (81.3)	-5.55 (18.69)	(-43.84, 32.74)				
Week 10	16			14 (87.5)	-21.36 (14.82)	(-51.66, 8.95)				
Week 12	16			14 (87.5)	-26.56 (12.72)	(-52.53, -0.59)				
Week 14	16			14 (87.5)	-17.06 (15.34)	(-48.39, 14.27)				
Week 16	16			13 (81.3)	-16.09 (16.54)	(-49.92, 17.74)				
Week 18	16			15 (93.8)	-13.22 (15.24)	(-44.22, 17.78)				
Week 20	16			14 (87.5)	-42.23 (11.40)	(-65.48, -18.98)				
Week 22	16			14 (87.5)	-39.98 (12.45)	(-65.37, -14.59)				
Week 24	16			14 (87.5)	-34.82 (12.17)	(-59.62, -10.03)				
Week 26	16	10 (62.5)	-32.49 (15.43)	(-64.26, -0.72)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.

ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.

ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to

Peg. NE = not evaluable. IA = p-value of interaction term.

ARC = Absolute reticulocyte counts (10**⁻⁹/l).

Source Data: aeфф, created on: 13SEP2023

Table PT1RCWC_SIHB: Change from baseline in ARC (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
ARC level (censored)	Latin America	Baseline	Peg	12	12 (100.0)	225.00 (73.05)	90.0	217.50	360.0	
			SoC	2	2 (100.0)	140.00 (63.64)	95.0	140.00	185.0	
		Week 2	Peg	12	9 (75.0)	81.11 (30.60)	40.0	80.00	140.0	
			SoC	2	1 (50.0)	140.00 (NE)	140.0	140.00	140.0	
		Week 4	Peg	12	9 (75.0)	72.22 (39.30)	20.0	60.00	160.0	
			SoC	2	1 (50.0)	100.00 (NE)	100.0	100.00	100.0	
		Week 6	Peg	12	7 (58.3)	55.71 (21.49)	30.0	50.00	80.0	
			SoC	2	1 (50.0)	150.00 (NE)	150.0	150.00	150.0	
		Week 8	Peg	12	4 (33.3)	70.00 (45.46)	20.0	65.00	130.0	
			SoC	2	1 (50.0)	150.00 (NE)	150.0	150.00	150.0	
		Week 10	Peg	12	3 (25.0)	70.00 (55.68)	20.0	60.00	130.0	
			SoC	2	2 (100.0)	167.50 (24.75)	150.0	167.50	185.0	
		Week 12	Peg	12	3 (25.0)	106.67 (28.87)	90.0	90.00	140.0	
			SoC	2	1 (50.0)	185.00 (NE)	185.0	185.00	185.0	
		Week 14	Peg	12	3 (25.0)	93.33 (61.10)	40.0	80.00	160.0	
			SoC	2	1 (50.0)	185.00 (NE)	185.0	185.00	185.0	
		Week 16	Peg	12	5 (41.7)	86.00 (47.22)	30.0	80.00	160.0	
			SoC	2	1 (50.0)	185.00 (NE)	185.0	185.00	185.0	
		Week 18	Peg	12	6 (50.0)	65.00 (26.65)	30.0	70.00	100.0	
			SoC	2	1 (50.0)	185.00 (NE)	185.0	185.00	185.0	
		Week 20	Peg	12	6 (50.0)	65.00 (36.74)	30.0	50.00	120.0	
			SoC	2	1 (50.0)	185.00 (NE)	185.0	185.00	185.0	
		Week 22	Peg	12	7 (58.3)	61.43 (33.88)	10.0	60.00	110.0	
			SoC	2	1 (50.0)	185.00 (NE)	185.0	185.00	185.0	
		Week 24	Peg	12	8 (66.7)	75.00 (42.76)	0.0	90.00	130.0	
			SoC	2	0 (0.0)	NE (NE)	NE	NE	NE	
		Week 26	Peg	12	7 (58.3)	98.57 (36.25)	40.0	100.00	140.0	
			SoC	2	1 (50.0)	185.00 (NE)	185.0	185.00	185.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10**9/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeFF, created on: 13SEP2023

Table PT1RCWC_SIHB: Change from baseline in ARC (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
ARC level (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	232.90 (86.29)	45.0	255.00	403.3	
			SoC	16	16 (100.0)	185.31 (113.86)	35.0	177.50	510.0	
	Week 2	Peg	23	22 (95.7)	90.00 (38.54)	20.0	75.00	190.0		
		SoC	16	15 (93.8)	176.67 (119.32)	20.0	160.00	500.0		
	Week 4	Peg	23	20 (87.0)	65.00 (26.06)	20.0	65.00	100.0		
		SoC	16	13 (81.3)	200.77 (125.40)	10.0	190.00	500.0		
	Week 6	Peg	23	20 (87.0)	77.00 (25.77)	40.0	70.00	150.0		
		SoC	16	14 (87.5)	183.57 (128.76)	20.0	170.00	530.0		
	Week 8	Peg	23	19 (82.6)	78.95 (29.98)	40.0	70.00	130.0		
		SoC	16	13 (81.3)	197.69 (139.47)	30.0	170.00	470.0		
	Week 10	Peg	23	19 (82.6)	89.47 (28.77)	20.0	80.00	130.0		
		SoC	16	14 (87.5)	184.29 (120.24)	30.0	150.00	460.0		
	Week 12	Peg	23	20 (87.0)	106.50 (28.15)	40.0	110.00	150.0		
		SoC	16	14 (87.5)	180.00 (107.20)	30.0	165.00	400.0		
	Week 14	Peg	23	20 (87.0)	109.50 (26.45)	70.0	110.00	170.0		
		SoC	16	14 (87.5)	176.43 (126.59)	30.0	150.00	500.0		
	Week 16	Peg	23	20 (87.0)	105.00 (30.00)	60.0	105.00	190.0		
		SoC	16	13 (81.3)	173.08 (135.12)	30.0	150.00	540.0		
	Week 18	Peg	23	22 (95.7)	97.27 (24.53)	50.0	95.00	160.0		
		SoC	16	15 (93.8)	180.67 (123.89)	30.0	160.00	480.0		
	Week 20	Peg	23	21 (91.3)	91.43 (22.42)	60.0	90.00	130.0		
		SoC	16	14 (87.5)	158.57 (97.73)	30.0	145.00	430.0		
	Week 22	Peg	23	21 (91.3)	92.86 (25.52)	50.0	100.00	130.0		
		SoC	16	14 (87.5)	161.43 (103.84)	30.0	145.00	430.0		
	Week 24	Peg	23	22 (95.7)	94.09 (25.01)	40.0	90.00	140.0		
		SoC	16	14 (87.5)	163.57 (101.27)	30.0	150.00	430.0		
	Week 26	Peg	23	19 (82.6)	102.11 (29.55)	60.0	100.00	190.0		
		SoC	16	10 (62.5)	174.00 (118.90)	30.0	170.00	430.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10**9/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeFF, created on: 13SEP2023

Table PT1RCWC_SIHB: Change from baseline in ARC (censored) by region
ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in ARC (censored)	Latin America	Week 2	Peg	12	9 (75.0)	-146.11 (100.02)	-320.0	-130.00	10.0	NE (NE) [NE, NE]
			SoC	2	1 (50.0)	45.00 (NE)	45.0	45.00	45.0	
		Week 4	Peg	12	9 (75.0)	-148.33 (92.84)	-320.0	-120.00	0.0	NE (NE) [NE, NE]
			SoC	2	1 (50.0)	5.00 (NE)	5.0	5.00	5.0	
		Week 6	Peg	12	7 (58.3)	-155.71 (105.53)	-310.0	-130.00	-20.0	NE (NE) [NE, NE]
			SoC	2	1 (50.0)	55.00 (NE)	55.0	55.00	55.0	
		Week 8	Peg	12	4 (33.3)	-165.00 (100.25)	-300.0	-142.50	-75.0	NE (NE) [NE, NE]
			SoC	2	1 (50.0)	55.00 (NE)	55.0	55.00	55.0	
		Week 10	Peg	12	3 (25.0)	-195.00 (98.36)	-300.0	-180.00	-105.0	-1.94 (2.58) [-8.45, -0.16]
			SoC	2	2 (100.0)	27.50 (38.89)	0.0	27.50	55.0	
		Week 12	Peg	12	3 (25.0)	-158.33 (97.00)	-270.0	-110.00	-95.0	NE (NE) [NE, NE]
			SoC	2	1 (50.0)	0.00 (NE)	0.0	0.00	0.0	
		Week 14	Peg	12	3 (25.0)	-171.67 (103.00)	-280.0	-160.00	-75.0	NE (NE) [NE, NE]
			SoC	2	1 (50.0)	0.00 (NE)	0.0	0.00	0.0	
		Week 16	Peg	12	5 (41.7)	-165.00 (73.82)	-270.0	-170.00	-75.0	NE (NE) [NE, NE]
			SoC	2	1 (50.0)	0.00 (NE)	0.0	0.00	0.0	
		Week 18	Peg	12	6 (50.0)	-140.00 (82.76)	-280.0	-137.50	-50.0	NE (NE) [NE, NE]
			SoC	2	1 (50.0)	0.00 (NE)	0.0	0.00	0.0	
		Week 20	Peg	12	6 (50.0)	-178.33 (91.14)	-300.0	-160.00	-50.0	NE (NE) [NE, NE]
			SoC	2	1 (50.0)	0.00 (NE)	0.0	0.00	0.0	
		Week 22	Peg	12	7 (58.3)	-152.86 (69.87)	-290.0	-145.00	-80.0	NE (NE) [NE, NE]
			SoC	2	1 (50.0)	0.00 (NE)	0.0	0.00	0.0	
		Week 24	Peg	12	8 (66.7)	-136.25 (69.94)	-270.0	-142.50	-55.0	NE (NE) [NE, NE]
			SoC	2	0 (0.0)	NE (NE)	NE	NE	NE	
		Week 26	Peg	12	7 (58.3)	-152.86 (67.69)	-270.0	-135.00	-50.0	NE (NE) [NE, NE]
			SoC	2	1 (50.0)	0.00 (NE)	0.0	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
ARC = Absolute reticulocyte counts (10**⁻⁹/l).
Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
Source Data: aeff, created on: 13SEP2023

Table PT1RCWC_SIHB: Change from baseline in ARC (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in ARC (censored)	Asia	Week 2	Peg	23	22 (95.7)	-141.89 (85.37)	-333.3	-140.00	25.0	-1.74 (0.41) [-2.65, -1.05]
			SoC	16	15 (93.8)	-14.67 (42.82)	-115.0	-5.00	55.0	
	Pacific	Week 4	Peg	23	20 (87.0)	-167.33 (92.88)	-303.3	-192.50	35.0	-2.15 (0.47) [-3.22, -1.38]
			SoC	16	13 (81.3)	7.31 (50.97)	-110.0	0.00	75.0	
	Week 6	Peg	23	20 (87.0)	-155.33 (85.43)	-303.3	-162.50	25.0	-1.96 (0.44) [-2.97, -1.23]	
		SoC	16	14 (87.5)	-1.43 (61.25)	-120.0	5.00	105.0		
	Week 8	Peg	23	19 (82.6)	-162.46 (80.57)	-273.3	-175.00	45.0	-2.23 (0.48) [-3.34, -1.45]	
		SoC	16	13 (81.3)	10.38 (67.25)	-60.0	-5.00	215.0		
	Week 10	Peg	23	19 (82.6)	-151.93 (76.28)	-283.3	-165.00	45.0	-2.15 (0.47) [-3.22, -1.39]	
		SoC	16	14 (87.5)	-6.43 (48.14)	-85.0	-7.50	115.0		
	Week 12	Peg	23	20 (87.0)	-137.08 (68.89)	-263.3	-155.00	35.0	-1.98 (0.45) [-2.99, -1.24]	
		SoC	16	14 (87.5)	-10.71 (51.14)	-110.0	-5.00	105.0		
	Week 14	Peg	23	20 (87.0)	-137.58 (73.08)	-303.3	-127.50	35.0	-2.07 (0.45) [-3.10, -1.32]	
		SoC	16	14 (87.5)	-1.07 (49.23)	-85.0	-7.50	135.0		
	Week 16	Peg	23	20 (87.0)	-142.08 (79.52)	-283.3	-160.00	45.0	-2.10 (0.47) [-3.16, -1.33]	
		SoC	16	13 (81.3)	0.00 (35.47)	-50.0	-5.00	75.0		
	Week 18	Peg	23	22 (95.7)	-137.80 (79.05)	-273.3	-165.00	45.0	-1.92 (0.42) [-2.86, -1.21]	
		SoC	16	15 (93.8)	1.67 (57.56)	-85.0	-5.00	165.0		
	Week 20	Peg	23	21 (91.3)	-140.79 (79.54)	-283.3	-160.00	35.0	-1.67 (0.42) [-2.60, -0.96]	
		SoC	16	14 (87.5)	-26.43 (40.21)	-85.0	-20.00	65.0		
Week 22	Peg	23	21 (91.3)	-141.27 (82.04)	-303.3	-160.00	45.0	-1.67 (0.42) [-2.61, -0.97]		
	SoC	16	14 (87.5)	-23.57 (40.31)	-85.0	-17.50	45.0			

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10**9/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1RCWC_SIHB: Change from baseline in ARC (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in ARC (censored)	Asia	Week 24	Peg	23	22 (95.7)	-140.98 (81.69)	-293.3	-167.50	35.0	-1.77 (0.42) [-2.71, -1.06]
			SoC	16	14 (87.5)	-15.00 (43.10)	-85.0	-5.00	65.0	
	Pacific	Week 26	Peg	23	19 (82.6)	-147.19 (79.58)	-283.3	-170.00	25.0	-1.97 (0.50) [-3.11, -1.15]
			SoC	16	10 (62.5)	-9.00 (36.27)	-80.0	-12.50	55.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10^{**}-9/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeFF, created on: 13SEP2023

Table PT1RCWC_SICA: Change from baseline in ARC (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.8928

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 ARC = Absolute reticulocyte counts (10^{**}-9/l).
 Source Data: aeфф, created on: 13SEP2023

Table PT1RCWC_SICA: Change from baseline in ARC (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 2	21	17 (81.0)	-151.88 (8.58)	(-169.77, -133.99)	-89.43 (17.64)	(-126.22, -52.64)	<0.0001	*
		Week 4	21	16 (76.2)	-173.75 (9.50)	(-193.62, -153.87)	-120.96 (19.12)	(-160.97, -80.94)	<0.0001	*
		Week 6	21	16 (76.2)	-162.15 (12.38)	(-188.06, -136.24)	-135.10 (24.92)	(-187.27, -82.94)	<0.0001	*
		Week 8	21	14 (66.7)	-171.58 (8.86)	(-190.37, -152.79)	-124.68 (18.40)	(-163.69, -85.68)	<0.0001	*
		Week 10	21	14 (66.7)	-158.67 (9.21)	(-178.10, -139.24)	-109.45 (17.95)	(-147.32, -71.58)	<0.0001	*
		Week 12	21	14 (66.7)	-136.26 (7.57)	(-152.23, -120.30)	-89.76 (14.75)	(-120.88, -58.64)	<0.0001	*
		Week 14	21	14 (66.7)	-134.69 (9.74)	(-155.14, -114.24)	-83.83 (18.59)	(-122.89, -44.77)	0.0003	*
		Week 16	21	16 (76.2)	-141.87 (9.74)	(-162.18, -121.55)	-83.03 (19.46)	(-123.62, -42.44)	0.0004	*
		Week 18	21	16 (76.2)	-142.52 (8.46)	(-160.10, -124.93)	-102.34 (15.82)	(-135.24, -69.44)	<0.0001	*
		Week 20	21	15 (71.4)	-151.37 (8.37)	(-168.90, -133.85)	-91.94 (15.82)	(-125.05, -58.82)	<0.0001	*
		Week 22	21	15 (71.4)	-151.52 (8.70)	(-169.72, -133.32)	-90.25 (16.31)	(-124.39, -56.11)	<0.0001	*
		Week 24	21	17 (81.0)	-145.71 (8.47)	(-163.38, -128.03)	-89.09 (17.86)	(-126.34, -51.85)	<0.0001	*
		Week 26	21	15 (71.4)	-145.63 (8.78)	(-164.15, -127.10)	-90.17 (19.10)	(-130.47, -49.87)	0.0002	*
		SoC	Week 2	8	6 (75.0)	-62.45 (14.91)	(-93.55, -31.35)			
	Week 4		8	6 (75.0)	-52.79 (16.01)	(-86.30, -19.28)				
	Week 6		8	6 (75.0)	-27.05 (20.87)	(-70.73, 16.64)				
	Week 8		8	5 (62.5)	-46.90 (15.46)	(-79.67, -14.13)				
	Week 10		8	6 (75.0)	-49.22 (14.62)	(-80.06, -18.38)				
	Week 12		8	6 (75.0)	-46.50 (12.01)	(-71.84, -21.16)				
	Week 14		8	7 (87.5)	-50.86 (14.49)	(-81.30, -20.42)				
	Week 16		8	7 (87.5)	-58.83 (15.59)	(-91.35, -26.32)				
	Week 18		8	8 (100.0)	-40.18 (12.45)	(-66.06, -14.29)				
	Week 20		8	7 (87.5)	-59.44 (12.70)	(-86.01, -32.87)				
	Week 22		8	7 (87.5)	-61.27 (13.13)	(-88.75, -33.79)				
	Week 24		8	6 (75.0)	-56.61 (14.98)	(-87.85, -25.37)				
	Week 26	8	5 (62.5)	-55.46 (16.11)	(-89.45, -21.47)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.

ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.

ANCOVA: fixed effects = treatment, covariate = baseline.

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to

Peg. NE = not evaluable. IA = p-value of interaction term.

ARC = Absolute reticulocyte counts (10⁹/l).

Source Data: aeфф, created on: 13SEP2023

Table PT1RCWC_SICA: Change from baseline in ARC (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA	
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value		
>= 4	Peg	Week 2	14	14 (100.0)	-111.47 (17.87)	(-148.63, -74.31)	-102.03 (27.82)	(-159.89, -44.16)	0.0014	*	
		Week 4	14	13 (92.9)	-127.15 (20.16)	(-169.50, -84.81)	-148.16 (32.75)	(-216.96, -79.35)	0.0003	*	
		Week 6	14	11 (78.6)	-123.40 (22.33)	(-170.51, -76.28)	-118.10 (33.42)	(-188.60, -47.60)	0.0025	*	
		Week 8	14	9 (64.3)	-123.40 (27.53)	(-182.07, -64.73)	-144.58 (39.27)	(-228.29, -60.87)	0.0022	*	
		Week 10	14	8 (57.1)	-123.92 (24.04)	(-175.15, -72.69)	-124.06 (32.56)	(-193.46, -54.66)	0.0017	*	
		Week 12	14	9 (64.3)	-114.97 (19.42)	(-156.36, -73.58)	-98.83 (27.72)	(-157.92, -39.75)	0.0028	*	
		Week 14	14	9 (64.3)	-115.67 (23.48)	(-166.03, -65.30)	-115.79 (34.73)	(-190.28, -41.31)	0.0049	*	
		Week 16	14	9 (64.3)	-118.46 (23.45)	(-169.13, -67.80)	-130.05 (36.11)	(-208.05, -52.05)	0.0032	*	
		Week 18	14	12 (85.7)	-109.12 (21.12)	(-153.69, -64.55)	-117.17 (33.46)	(-187.77, -46.57)	0.0027	*	
		Week 20	14	12 (85.7)	-121.44 (15.51)	(-154.16, -88.73)	-89.86 (24.64)	(-141.83, -37.88)	0.0020	*	
		Week 22	14	13 (92.9)	-115.58 (14.20)	(-145.42, -85.74)	-95.26 (23.09)	(-143.76, -46.76)	0.0006	*	
		Week 24	14	13 (92.9)	-108.62 (13.50)	(-136.99, -80.26)	-87.01 (21.94)	(-133.11, -40.92)	0.0009	*	
		Week 26	14	11 (78.6)	-125.31 (17.02)	(-161.81, -88.81)	-105.87 (29.10)	(-168.30, -43.45)	0.0027	*	
			SoC	Week 2	10	10 (100.0)	-9.44 (21.17)	(-53.47, 34.59)			
		Week 4		10	8 (80.0)	21.00 (25.72)	(-33.04, 75.04)				
		Week 6		10	9 (90.0)	-5.29 (24.70)	(-57.41, 46.83)				
		Week 8		10	9 (90.0)	21.18 (27.53)	(-37.49, 79.85)				
		Week 10		10	10 (100.0)	0.14 (21.45)	(-45.57, 45.85)				
		Week 12		10	9 (90.0)	-16.14 (19.42)	(-57.53, 25.25)				
		Week 14		10	8 (80.0)	0.13 (24.95)	(-53.38, 53.63)				
		Week 16		10	7 (70.0)	11.59 (26.70)	(-46.09, 69.28)				
		Week 18		10	8 (80.0)	8.05 (25.89)	(-46.57, 62.67)				
		Week 20		10	8 (80.0)	-31.59 (19.02)	(-71.72, 8.55)				
		Week 22		10	8 (80.0)	-20.31 (18.13)	(-58.40, 17.77)				
		Week 24		10	8 (80.0)	-21.61 (17.23)	(-57.81, 14.59)				
		Week 26	10	6 (60.0)	-19.44 (23.21)	(-69.22, 30.35)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.

ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.

ANCOVA: fixed effects = treatment, covariate = baseline.

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to

Peg. NE = not evaluable. IA = p-value of interaction term.

ARC = Absolute reticulocyte counts (10⁹/l).

Source Data: aeфф, created on: 13SEP2023

Table PT1RCWC_SIHA: Change from baseline in ARC (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
ARC level (censored) < 4	Baseline	Peg	21	21 (100.0)	238.89 (67.91)	130.0	255.00	403.3	
		SoC	8	8 (100.0)	172.50 (58.49)	85.0	185.00	260.0	
	Week 2	Peg	21	17 (81.0)	76.47 (30.40)	20.0	70.00	150.0	
		SoC	8	6 (75.0)	158.33 (47.08)	70.0	165.00	210.0	
	Week 4	Peg	21	16 (76.2)	56.25 (25.53)	20.0	50.00	100.0	
		SoC	8	6 (75.0)	163.33 (66.53)	70.0	180.00	240.0	
	Week 6	Peg	21	16 (76.2)	68.75 (29.41)	30.0	70.00	150.0	
		SoC	8	6 (75.0)	186.67 (89.81)	80.0	205.00	320.0	
	Week 8	Peg	21	14 (66.7)	65.71 (31.31)	20.0	60.00	130.0	
		SoC	8	5 (62.5)	168.00 (58.48)	80.0	170.00	230.0	
	Week 10	Peg	21	14 (66.7)	77.86 (35.56)	20.0	80.00	130.0	
		SoC	8	6 (75.0)	162.50 (54.20)	70.0	167.50	230.0	
	Week 12	Peg	21	14 (66.7)	99.29 (29.99)	40.0	90.00	150.0	
		SoC	8	6 (75.0)	167.50 (45.36)	90.0	170.00	230.0	
	Week 14	Peg	21	14 (66.7)	100.00 (32.58)	40.0	95.00	170.0	
		SoC	8	7 (87.5)	157.86 (51.95)	90.0	170.00	230.0	
	Week 16	Peg	21	16 (76.2)	92.50 (35.87)	30.0	85.00	190.0	
		SoC	8	7 (87.5)	149.29 (54.80)	90.0	150.00	230.0	
	Week 18	Peg	21	16 (76.2)	88.13 (31.46)	30.0	90.00	160.0	
		SoC	8	8 (100.0)	163.13 (55.74)	70.0	177.50	230.0	
	Week 20	Peg	21	15 (71.4)	82.67 (25.76)	30.0	90.00	130.0	
		SoC	8	7 (87.5)	156.43 (52.97)	70.0	140.00	230.0	
	Week 22	Peg	21	15 (71.4)	80.00 (26.99)	30.0	70.00	130.0	
		SoC	8	7 (87.5)	152.14 (55.67)	70.0	140.00	230.0	
	Week 24	Peg	21	17 (81.0)	82.35 (26.58)	30.0	90.00	130.0	
		SoC	8	6 (75.0)	153.33 (60.22)	70.0	155.00	230.0	
	Week 26	Peg	21	15 (71.4)	90.00 (26.19)	40.0	90.00	140.0	
		SoC	8	5 (62.5)	155.00 (67.08)	70.0	185.00	230.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10**⁻⁹/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeFF, created on: 13SEP2023

Table PT1RCWC_SIHA: Change from baseline in ARC (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
ARC level (censored) >= 4	Baseline	Peg	14	14 (100.0)	217.14 (98.76)	45.0	252.50	320.0	
		SoC	10	10 (100.0)	186.50 (140.38)	35.0	157.50	510.0	
	Week 2	Peg	14	14 (100.0)	100.71 (39.12)	50.0	100.00	190.0	
		SoC	10	10 (100.0)	184.00 (144.16)	20.0	145.00	500.0	
	Week 4	Peg	14	13 (92.9)	80.77 (30.95)	30.0	80.00	160.0	
		SoC	10	8 (80.0)	216.25 (154.17)	10.0	180.00	500.0	
	Week 6	Peg	14	11 (78.6)	75.45 (21.15)	30.0	70.00	120.0	
		SoC	10	9 (90.0)	177.78 (148.31)	20.0	150.00	530.0	
	Week 8	Peg	14	9 (64.3)	95.56 (25.06)	50.0	90.00	130.0	
		SoC	10	9 (90.0)	208.89 (164.50)	30.0	150.00	470.0	
	Week 10	Peg	14	8 (57.1)	102.50 (19.09)	80.0	100.00	130.0	
		SoC	10	10 (100.0)	194.00 (137.70)	30.0	150.00	460.0	
	Week 12	Peg	14	9 (64.3)	117.78 (19.86)	80.0	120.00	140.0	
		SoC	10	9 (90.0)	188.89 (131.10)	30.0	150.00	400.0	
	Week 14	Peg	14	9 (64.3)	118.89 (26.67)	80.0	110.00	160.0	
		SoC	10	8 (80.0)	193.75 (163.61)	30.0	150.00	500.0	
	Week 16	Peg	14	9 (64.3)	116.67 (24.49)	90.0	110.00	160.0	
		SoC	10	7 (70.0)	198.57 (179.20)	30.0	150.00	540.0	
	Week 18	Peg	14	12 (85.7)	93.33 (23.48)	40.0	100.00	120.0	
		SoC	10	8 (80.0)	198.75 (163.92)	30.0	150.00	480.0	
	Week 20	Peg	14	12 (85.7)	89.17 (30.88)	40.0	100.00	130.0	
		SoC	10	8 (80.0)	163.75 (124.09)	30.0	150.00	430.0	
	Week 22	Peg	14	13 (92.9)	90.77 (34.27)	10.0	90.00	130.0	
		SoC	10	8 (80.0)	172.50 (131.23)	30.0	150.00	430.0	
	Week 24	Peg	14	13 (92.9)	97.69 (35.39)	0.0	110.00	140.0	
		SoC	10	8 (80.0)	171.25 (127.66)	30.0	150.00	430.0	
	Week 26	Peg	14	11 (78.6)	116.36 (31.07)	70.0	110.00	190.0	
		SoC	10	6 (60.0)	191.67 (145.38)	30.0	175.00	430.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10**⁻⁹/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1RCWC_SIHA: Change from baseline in ARC (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in ARC (censored)	Week 2	Peg	21	17 (81.0)	-165.10 (77.05)	-333.3	-140.00	-63.3	-1.93 (0.60) [-3.31, -0.96]
		SoC	8	6 (75.0)	-25.00 (40.25)	-90.0	-17.50	25.0	
	Week 4	Peg	21	16 (76.2)	-186.04 (69.15)	-320.0	-185.00	-80.0	-2.45 (0.66) [-4.02, -1.43]
		SoC	8	6 (75.0)	-20.00 (51.87)	-110.0	-17.50	50.0	
	Week 6	Peg	21	16 (76.2)	-173.54 (70.03)	-310.0	-162.50	-60.0	-2.47 (0.66) [-4.04, -1.44]
		SoC	8	6 (75.0)	3.33 (65.85)	-120.0	17.50	60.0	
	Week 8	Peg	21	14 (66.7)	-184.05 (65.33)	-300.0	-177.50	-70.0	-2.77 (0.77) [-4.64, -1.63]
		SoC	8	5 (62.5)	-12.00 (33.65)	-60.0	-5.00	25.0	
	Week 10	Peg	21	14 (66.7)	-171.90 (64.80)	-300.0	-170.00	-80.0	-2.60 (0.71) [-4.32, -1.54]
		SoC	8	6 (75.0)	-18.33 (23.80)	-60.0	-12.50	5.0	
	Week 12	Peg	21	14 (66.7)	-150.48 (64.73)	-270.0	-152.50	-40.0	-2.33 (0.68) [-3.95, -1.30]
		SoC	8	6 (75.0)	-13.33 (23.17)	-50.0	-7.50	10.0	
	Week 14	Peg	21	14 (66.7)	-154.76 (70.89)	-303.3	-145.00	-60.0	-2.31 (0.64) [-3.85, -1.33]
		SoC	8	7 (87.5)	-10.71 (21.10)	-40.0	-10.00	25.0	
	Week 16	Peg	21	16 (76.2)	-159.17 (68.86)	-283.3	-177.50	-50.0	-2.28 (0.61) [-3.72, -1.32]
		SoC	8	7 (87.5)	-19.29 (20.30)	-50.0	-20.00	5.0	
	Week 18	Peg	21	16 (76.2)	-157.92 (62.91)	-280.0	-165.00	-63.3	-2.67 (0.63) [-4.17, -1.70]
		SoC	8	8 (100.0)	-9.38 (24.70)	-40.0	-10.00	40.0	
	Week 20	Peg	21	15 (71.4)	-166.44 (68.01)	-300.0	-170.00	-50.0	-2.28 (0.63) [-3.76, -1.31]
		SoC	8	7 (87.5)	-27.14 (26.75)	-70.0	-20.00	0.0	
Week 22	Peg	21	15 (71.4)	-165.44 (68.14)	-303.3	-170.00	-53.3	-2.18 (0.62) [-3.64, -1.22]	
	SoC	8	7 (87.5)	-31.43 (27.80)	-70.0	-30.00	0.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10**⁻⁹/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1RCWC_SIHA: Change from baseline in ARC (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in ARC (censored)	Week 24	Peg	21	17 (81.0)	-160.39 (69.49)	-293.3	-170.00	-50.0	-2.20 (0.62) [-3.66, -1.22]
		SoC	8	6 (75.0)	-15.00 (38.99)	-80.0	-12.50	25.0	
	Week 26	Peg	21	15 (71.4)	-160.11 (68.39)	-283.3	-160.00	-50.0	-2.33 (0.70) [-3.99, -1.25]
		SoC	8	5 (62.5)	-12.00 (18.91)	-30.0	-20.00	15.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10**⁻⁹/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1RCWC_SIHA: Change from baseline in ARC (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in ARC (censored)	Week 2	Peg	14	14 (100.0)	-116.43 (96.09)	-270.0	-130.00	25.0	-1.38 (0.49) [-2.49, -0.56]
		SoC	10	10 (100.0)	-2.50 (45.96)	-115.0	-2.50	55.0	
	Week 4	Peg	14	13 (92.9)	-131.15 (108.69)	-290.0	-145.00	35.0	-1.70 (0.57) [-3.02, -0.80]
		SoC	10	8 (80.0)	27.50 (37.80)	-25.0	22.50	75.0	
	Week 6	Peg	14	11 (78.6)	-129.09 (109.11)	-275.0	-125.00	25.0	-1.38 (0.54) [-2.62, -0.49]
		SoC	10	9 (90.0)	1.67 (61.29)	-85.0	10.00	105.0	
	Week 8	Peg	14	9 (64.3)	-130.00 (97.18)	-270.0	-115.00	45.0	-1.72 (0.61) [-3.17, -0.77]
		SoC	10	9 (90.0)	27.78 (76.24)	-40.0	5.00	215.0	
	Week 10	Peg	14	8 (57.1)	-133.13 (97.58)	-240.0	-150.00	45.0	-1.74 (0.62) [-3.20, -0.78]
		SoC	10	10 (100.0)	7.50 (56.09)	-85.0	0.00	115.0	
	Week 12	Peg	14	9 (64.3)	-123.33 (80.35)	-220.0	-155.00	35.0	-1.53 (0.59) [-2.91, -0.59]
		SoC	10	9 (90.0)	-7.78 (62.56)	-110.0	-5.00	105.0	
	Week 14	Peg	14	9 (64.3)	-122.22 (82.62)	-220.0	-125.00	35.0	-1.66 (0.63) [-3.15, -0.69]
		SoC	10	8 (80.0)	7.50 (62.79)	-85.0	-5.00	135.0	
	Week 16	Peg	14	9 (64.3)	-124.44 (90.81)	-230.0	-135.00	45.0	-1.88 (0.68) [-3.53, -0.85]
		SoC	10	7 (70.0)	19.29 (35.17)	-20.0	5.00	75.0	
	Week 18	Peg	14	12 (85.7)	-112.08 (91.32)	-240.0	-120.00	45.0	-1.39 (0.55) [-2.65, -0.49]
		SoC	10	8 (80.0)	12.50 (75.78)	-85.0	-5.00	165.0	
	Week 20	Peg	14	12 (85.7)	-127.50 (95.24)	-265.0	-142.50	35.0	-1.25 (0.54) [-2.47, -0.35]
		SoC	10	8 (80.0)	-22.50 (49.71)	-85.0	-12.50	65.0	
Week 22	Peg	14	13 (92.9)	-119.62 (84.15)	-240.0	-145.00	45.0	-1.40 (0.54) [-2.62, -0.51]	
	SoC	10	8 (80.0)	-13.75 (47.57)	-85.0	-5.00	45.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10**9/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1RCWC_SIHA: Change from baseline in ARC (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in ARC (censored)	Week 24	Peg	14	13 (92.9)	-112.69 (81.89)	-220.0	-145.00	35.0	-1.31 (0.53) [-2.52, -0.43]
		SoC	10	8 (80.0)	-15.00 (48.62)	-85.0	-5.00	65.0	
	Week 26	Peg	14	11 (78.6)	-133.18 (84.60)	-210.0	-145.00	25.0	-1.65 (0.64) [-3.17, -0.64]
		SoC	10	6 (60.0)	-5.00 (45.50)	-80.0	-5.00	55.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 ARC = Absolute reticulocyte counts (10**⁻⁹/l).
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: aeff, created on: 13SEP2023

Table PT1TWT_SITC: Time to transfusion (censored) by age
 ITT

Time to transfusion (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 >= 3 consecutive visits missed: at last visit before the streak. No baseline value or no post-baseline value or
 if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake. * = significant
 treatment effect. IA = p-value of interaction term.
 Source Data: atf_tte, created on: 18SEP2023

Table PT1TWT_SITC: Time to transfusion (censored) by age
 ITT

Time to transfusion (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	2 (6.9)	NE	(NE, NE)	0.03	(0.00, 0.15)	<0.0001	*
	SoC	13	9 (69.2)	9.43	(2.29, 10.86)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 >= 3 consecutive visits missed: at last visit before the streak. No baseline value or no post-baseline value or
 if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake. * = significant
 treatment effect. IA = p-value of interaction term.
 Source Data: atf_tte, created on: 18SEP2023

Table PT1TWT_SITC: Time to transfusion (censored) by age
 ITT

Time to transfusion (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	0 (0.0)	NE	(NE, NE)	NE	(NE, NE)	NE	
	SoC	5	4 (80.0)	7.00	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 >= 3 consecutive visits missed: at last visit before the streak. No baseline value or no post-baseline value or
 if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake. * = significant
 treatment effect. IA = p-value of interaction term.
 Source Data: atf_tte, created on: 18SEP2023

Table PT1TWT_SITB: Time to transfusion (censored) by region
 ITT

Time to transfusion (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 >= 3 consecutive visits missed: at last visit before the streak. No baseline value or no post-baseline value or
 if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake. * = significant
 treatment effect. IA = p-value of interaction term.
 Source Data: atf_tte, created on: 18SEP2023

Table PT1TWT_SITB: Time to transfusion (censored) by region
 ITT

Time to transfusion (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	2 (16.7)	NE	(17.71, NE)	NE	(NE, NE)	NE	
	SoC	2	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 >= 3 consecutive visits missed: at last visit before the streak. No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake. * = significant treatment effect. IA = p-value of interaction term.
 Source Data: atf_tte, created on: 18SEP2023

Table PT1TWT_SITB: Time to transfusion (censored) by region
ITT

Time to transfusion (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	0 (0.0)	NE	(NE, NE)	NE	(NE, NE)	NE	
	SoC	16	13 (81.3)	6.29	(3.14, 10.29)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
>= 3 consecutive visits missed: at last visit before the streak. No baseline value or no post-baseline value or
if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake. * = significant
treatment effect. IA = p-value of interaction term.
Source Data: atf_tte, created on: 18SEP2023

Table PT1TWT_SITA: Time to transfusion (censored) by number of transfusions previous year
 ITT

Time to transfusion (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 >= 3 consecutive visits missed: at last visit before the streak. No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake. * = significant treatment effect. IA = p-value of interaction term.
 Source Data: atf_tte, created on: 18SEP2023

Table PT1TWT_SITA: Time to transfusion (censored) by number of transfusions previous year
 ITT

Time to transfusion (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	0 (0.0)	NE	(NE, NE)	NE	(NE, NE)	NE	
	SoC	8	4 (50.0)	10.86	(3.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 >= 3 consecutive visits missed: at last visit before the streak. No baseline value or no post-baseline value or
 if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake. * = significant
 treatment effect. IA = p-value of interaction term.
 Source Data: atf_tte, created on: 18SEP2023

Table PT1TWT_SITA: Time to transfusion (censored) by number of transfusions previous year
 ITT

Time to transfusion (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	2 (14.3)	NE	(NE, NE)	0.03	(0.00, 0.23)	0.0009	*
	SoC	10	9 (90.0)	4.79	(0.86, 9.43)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 >= 3 consecutive visits missed: at last visit before the streak. No baseline value or no post-baseline value or
 if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake. * = significant
 treatment effect. IA = p-value of interaction term.
 Source Data: atf_tte, created on: 18SEP2023

Table PT1FAWC_SICC: Change from baseline in FACIT Fatigue score (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.8129

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SICC: Change from baseline in FACIT Fatigue score (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
< 60	Peg	Week 4	29	26 (89.7)	7.87 (1.32)	(5.19, 10.56)	9.53 (2.47)	(4.50, 14.56)	0.0005	*
		Week 8	29	24 (82.8)	7.75 (1.68)	(4.33, 11.18)	9.96 (3.06)	(3.72, 16.20)	0.0027	*
		Week 12	29	26 (89.7)	8.88 (1.42)	(6.00, 11.76)	10.55 (2.64)	(5.17, 15.93)	0.0003	*
		Week 16	29	25 (86.2)	8.44 (1.43)	(5.52, 11.35)	9.83 (2.54)	(4.65, 15.01)	0.0005	*
		Week 20	29	27 (93.1)	8.29 (1.55)	(5.14, 11.45)	9.47 (2.94)	(3.50, 15.43)	0.0028	*
		Week 26	29	23 (79.3)	9.58 (1.59)	(6.34, 12.82)	8.79 (2.84)	(2.99, 14.60)	0.0042	*
	SoC	Week 4	13	11 (84.6)	-1.65 (2.05)	(-5.82, 2.52)				
		Week 8	13	11 (84.6)	-2.21 (2.50)	(-7.30, 2.89)				
		Week 12	13	11 (84.6)	-1.66 (2.19)	(-6.12, 2.80)				
		Week 16	13	12 (92.3)	-1.39 (2.08)	(-5.61, 2.83)				
		Week 20	13	11 (84.6)	-1.17 (2.46)	(-6.17, 3.82)				
		Week 26	13	11 (84.6)	0.79 (2.32)	(-3.94, 5.52)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SICC: Change from baseline in FACIT Fatigue score (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	4.78 (2.64)	(-2.00, 11.57)	15.77 (4.56)	(4.06, 27.49)	0.0180	*
		Week 8	6	5 (83.3)	5.34 (1.99)	(0.22, 10.45)	10.30 (3.44)	(1.46, 19.14)	0.0302	*
		Week 12	6	5 (83.3)	3.75 (2.54)	(-2.78, 10.27)	9.47 (4.38)	(-1.80, 20.74)	0.0833	
		Week 16	6	5 (83.3)	6.81 (1.89)	(1.95, 11.67)	8.82 (3.15)	(0.73, 16.91)	0.0378	*
		Week 20	6	5 (83.3)	7.16 (2.08)	(1.82, 12.51)	10.55 (3.46)	(1.65, 19.45)	0.0285	*
	Week 26	6	5 (83.3)	5.92 (1.54)	(1.03, 10.82)	6.15 (3.66)	(-5.49, 17.78)	0.1914		
	SoC	Week 4	5	4 (80.0)	-10.99 (3.09)	(-18.94, -3.04)				
		Week 8	5	4 (80.0)	-4.97 (2.33)	(-10.96, 1.03)				
		Week 12	5	4 (80.0)	-5.72 (2.98)	(-13.37, 1.93)				
		Week 16	5	4 (80.0)	-2.01 (2.24)	(-7.76, 3.74)				
		Week 20	5	4 (80.0)	-3.39 (2.46)	(-9.72, 2.94)				
Week 26	5	2 (40.0)	-0.22 (3.03)	(-9.86, 9.42)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SIHC: Change from baseline in FACIT Fatigue score (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
FACIT Fatigue score (censored)	< 60	Baseline	Peg	29	29 (100.0)	35.31 (10.83)	12.0	39.00	52.0	
			SoC	13	12 (92.3)	39.08 (8.49)	20.0	40.00	49.0	
		Week 4	Peg	29	26 (89.7)	43.85 (6.47)	29.0	45.00	52.0	
			SoC	13	12 (92.3)	36.50 (9.33)	23.0	39.00	48.0	
		Week 8	Peg	29	24 (82.8)	43.88 (8.45)	16.0	46.50	51.0	
			SoC	13	11 (84.6)	35.27 (9.58)	18.0	39.00	45.0	
		Week 12	Peg	29	26 (89.7)	45.00 (8.03)	20.0	47.50	52.0	
			SoC	13	11 (84.6)	36.09 (8.24)	23.0	39.00	48.0	
		Week 16	Peg	29	25 (86.2)	45.08 (7.60)	20.0	47.00	52.0	
			SoC	13	12 (92.3)	36.58 (8.86)	23.0	38.00	48.0	
		Week 20	Peg	29	27 (93.1)	44.59 (8.61)	15.0	47.00	52.0	
			SoC	13	12 (92.3)	37.00 (8.26)	23.0	40.00	47.0	
		Week 26	Peg	29	23 (79.3)	45.22 (7.75)	20.0	48.00	52.0	
			SoC	13	11 (84.6)	37.73 (8.91)	23.0	39.00	50.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SIHC: Change from baseline in FACIT Fatigue score (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
FACIT Fatigue score (censored)	>= 60	Baseline	Peg	6	6 (100.0)	41.33 (8.98)	28.0	44.00	50.0	
			SoC	5	4 (80.0)	31.25 (10.44)	16.0	35.50	38.0	
	Week 4	Peg	6	5 (83.3)	46.80 (5.76)	38.0	50.00	52.0		
		SoC	5	5 (100.0)	26.80 (14.32)	7.0	28.00	47.0		
	Week 8	Peg	6	5 (83.3)	46.00 (3.54)	43.0	45.00	52.0		
		SoC	5	5 (100.0)	34.00 (9.95)	21.0	33.00	49.0		
	Week 12	Peg	6	5 (83.3)	44.20 (4.76)	40.0	43.00	52.0		
		SoC	5	5 (100.0)	33.60 (10.04)	21.0	33.00	49.0		
	Week 16	Peg	6	5 (83.3)	44.80 (5.89)	37.0	46.00	52.0		
		SoC	5	5 (100.0)	34.20 (9.93)	21.0	34.00	49.0		
	Week 20	Peg	6	5 (83.3)	45.00 (5.24)	40.0	43.00	52.0		
		SoC	5	5 (100.0)	33.60 (10.04)	21.0	33.00	49.0		
	Week 26	Peg	6	5 (83.3)	45.60 (5.03)	38.0	46.00	52.0		
		SoC	5	3 (60.0)	38.67 (8.96)	33.0	34.00	49.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SIHC: Change from baseline in FACIT Fatigue score (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in FACIT Fatigue score (censored)	Week 4	Peg	29	26 (89.7)	8.42 (8.75)	-8.0	7.50	26.0	1.27 (0.40) [0.56, 2.15]
		SoC	13	11 (84.6)	-3.00 (8.83)	-20.0	-1.00	12.0	
	Week 8	Peg	29	24 (82.8)	8.42 (9.74)	-12.0	7.00	28.0	1.23 (0.41) [0.51, 2.11]
		SoC	13	11 (84.6)	-3.64 (9.30)	-20.0	-4.00	12.0	
	Week 12	Peg	29	26 (89.7)	9.35 (9.04)	-8.0	8.00	28.0	1.32 (0.41) [0.61, 2.20]
		SoC	13	11 (84.6)	-2.82 (8.95)	-20.0	-1.00	12.0	
	Week 16	Peg	29	25 (86.2)	8.96 (9.02)	-8.0	8.00	28.0	1.26 (0.40) [0.57, 2.12]
		SoC	13	12 (92.3)	-2.50 (8.55)	-20.0	-1.00	12.0	
	Week 20	Peg	29	27 (93.1)	8.81 (10.50)	-13.0	8.00	29.0	1.10 (0.39) [0.40, 1.95]
		SoC	13	11 (84.6)	-2.45 (8.52)	-20.0	-3.00	12.0	
	Week 26	Peg	29	23 (79.3)	10.30 (9.88)	-8.0	9.00	29.0	1.11 (0.41) [0.39, 1.99]
		SoC	13	11 (84.6)	-0.73 (9.31)	-20.0	1.00	12.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SIHC: Change from baseline in FACIT Fatigue score (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in FACIT Fatigue score (censored)	Week 4	Peg	6	5 (83.3)	2.80 (8.29)	-4.0	1.00	17.0	1.60 (0.99) [0.29, 4.13]
		SoC	5	4 (80.0)	-9.50 (4.12)	-15.0	-9.00	-5.0	
	Week 8	Peg	6	5 (83.3)	2.00 (7.11)	-6.0	2.00	13.0	0.43 (0.81) [-1.01, 2.22]
		SoC	5	4 (80.0)	-1.00 (4.55)	-5.0	-2.00	5.0	
	Week 12	Peg	6	5 (83.3)	0.20 (7.98)	-9.0	1.00	12.0	0.22 (0.80) [-1.29, 1.90]
		SoC	5	4 (80.0)	-1.50 (5.20)	-7.0	-2.00	5.0	
	Week 16	Peg	6	5 (83.3)	5.00 (10.93)	-5.0	2.00	18.0	0.59 (0.82) [-0.82, 2.45]
		SoC	5	4 (80.0)	-0.75 (4.27)	-4.0	-2.00	5.0	
	Week 20	Peg	6	5 (83.3)	5.20 (9.09)	-6.0	2.00	16.0	0.78 (0.84) [-0.59, 2.75]
		SoC	5	4 (80.0)	-1.50 (5.20)	-7.0	-2.00	5.0	
	Week 26	Peg	6	5 (83.3)	5.80 (10.35)	-8.0	3.00	18.0	0.70 (1.14) [-1.12, 3.36]
		SoC	5	2 (40.0)	-2.00 (2.83)	-4.0	-2.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SICD: Change from baseline in FACIT Fatigue score (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.7447

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SICD: Change from baseline in FACIT Fatigue score (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Male	Peg	Week 4	19	16 (84.2)	5.68 (1.67)	(2.20, 9.17)	11.88 (2.88)	(5.88, 17.89)	0.0005	*
		Week 8	19	15 (78.9)	3.99 (2.00)	(-0.21, 8.18)	9.03 (3.41)	(1.90, 16.16)	0.0158	*
		Week 12	19	17 (89.5)	4.55 (1.62)	(1.19, 7.91)	8.62 (2.86)	(2.67, 14.58)	0.0067	*
		Week 16	19	16 (84.2)	5.66 (1.60)	(2.34, 8.97)	9.96 (2.69)	(4.36, 15.56)	0.0013	*
		Week 20	19	17 (89.5)	5.38 (1.93)	(1.36, 9.39)	8.63 (3.42)	(1.50, 15.75)	0.0199	*
		Week 26	19	15 (78.9)	6.91 (1.88)	(2.95, 10.87)	7.83 (3.34)	(0.82, 14.84)	0.0306	*
	SoC	Week 4	10	8 (80.0)	-6.20 (2.37)	(-11.15, -1.25)				
		Week 8	10	8 (80.0)	-5.05 (2.77)	(-10.84, 0.75)				
		Week 12	10	8 (80.0)	-4.07 (2.37)	(-9.01, 0.87)				
		Week 16	10	9 (90.0)	-4.30 (2.17)	(-8.82, 0.21)				
		Week 20	10	8 (80.0)	-3.25 (2.84)	(-9.16, 2.66)				
		Week 26	10	7 (70.0)	-0.92 (2.76)	(-6.72, 4.89)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SICD: Change from baseline in FACIT Fatigue score (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Female	Peg	Week 4	16	15 (93.8)	9.30 (2.07)	(4.95, 13.65)	12.41 (4.44)	(3.08, 21.74)	0.0120	*
		Week 8	16	14 (87.5)	10.47 (1.70)	(6.88, 14.06)	10.47 (3.70)	(2.66, 18.27)	0.0116	*
		Week 12	16	14 (87.5)	11.76 (1.70)	(8.18, 15.35)	12.13 (3.70)	(4.33, 19.93)	0.0044	*
		Week 16	16	14 (87.5)	11.00 (1.92)	(6.96, 15.04)	10.16 (4.17)	(1.37, 18.95)	0.0260	*
		Week 20	16	15 (93.8)	11.25 (1.59)	(7.91, 14.59)	12.35 (3.41)	(5.19, 19.51)	0.0019	*
		Week 26	16	13 (81.3)	10.54 (1.73)	(6.85, 14.23)	7.63 (4.02)	(-0.94, 16.20)	0.0772	
	SoC	Week 4	8	7 (87.5)	-3.10 (3.68)	(-10.82, 4.62)				
		Week 8	8	7 (87.5)	0.00 (2.95)	(-6.23, 6.23)				
		Week 12	8	7 (87.5)	-0.37 (2.95)	(-6.60, 5.85)				
		Week 16	8	7 (87.5)	0.84 (3.32)	(-6.18, 7.85)				
		Week 20	8	7 (87.5)	-1.10 (2.82)	(-7.03, 4.82)				
		Week 26	8	6 (75.0)	2.91 (3.24)	(-4.00, 9.82)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SIHD: Change from baseline in FACIT Fatigue score (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
FACIT Fatigue score (censored)	Male	Baseline	Peg	19	19 (100.0)	37.95 (10.58)	19.0	41.00	52.0	
			SoC	10	9 (90.0)	37.44 (8.00)	20.0	38.00	47.0	
		Week 4	Peg	19	16 (84.2)	43.31 (7.48)	29.0	46.00	52.0	
			SoC	10	9 (90.0)	33.00 (9.58)	23.0	29.00	48.0	
		Week 8	Peg	19	15 (78.9)	42.40 (10.14)	16.0	44.00	52.0	
			SoC	10	9 (90.0)	34.44 (9.51)	18.0	34.00	49.0	
		Week 12	Peg	19	17 (89.5)	42.82 (9.53)	20.0	44.00	52.0	
			SoC	10	9 (90.0)	35.11 (7.61)	25.0	34.00	49.0	
		Week 16	Peg	19	16 (84.2)	43.63 (9.00)	20.0	46.50	52.0	
			SoC	10	10 (100.0)	35.40 (7.76)	26.0	34.00	49.0	
		Week 20	Peg	19	17 (89.5)	42.29 (10.31)	15.0	45.00	52.0	
			SoC	10	9 (90.0)	35.67 (8.29)	25.0	34.00	49.0	
		Week 26	Peg	19	15 (78.9)	43.67 (9.48)	20.0	46.00	52.0	
			SoC	10	8 (80.0)	37.38 (8.53)	28.0	36.50	50.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SIHD: Change from baseline in FACIT Fatigue score (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
FACIT Fatigue score (censored)	Female	Baseline	Peg	16	16 (100.0)	34.44 (10.78)	12.0	37.50	47.0	
			SoC	8	7 (87.5)	36.71 (11.47)	16.0	39.00	49.0	
		Week 4	Peg	16	15 (93.8)	45.40 (4.94)	35.0	45.00	52.0	
			SoC	8	8 (100.0)	34.38 (13.98)	7.0	40.50	47.0	
		Week 8	Peg	16	14 (87.5)	46.21 (3.56)	39.0	46.50	50.0	
			SoC	8	7 (87.5)	35.43 (9.93)	21.0	41.00	45.0	
		Week 12	Peg	16	14 (87.5)	47.36 (2.73)	40.0	47.50	51.0	
			SoC	8	7 (87.5)	35.57 (10.33)	21.0	39.00	48.0	
		Week 16	Peg	16	14 (87.5)	46.64 (4.34)	37.0	47.50	51.0	
			SoC	8	7 (87.5)	36.57 (11.03)	21.0	42.00	48.0	
		Week 20	Peg	16	15 (93.8)	47.33 (3.11)	42.0	47.00	52.0	
			SoC	8	8 (100.0)	36.38 (9.58)	21.0	41.50	45.0	
		Week 26	Peg	16	13 (81.3)	47.15 (2.58)	42.0	48.00	50.0	
			SoC	8	6 (75.0)	38.67 (9.40)	23.0	41.50	50.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SIHD: Change from baseline in FACIT Fatigue score (censored) by sex
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Male in FACIT Fatigue score (censored)	Week 4	Peg	19	16 (84.2)	5.69 (8.92)	-8.0	4.00	23.0	1.31 (0.50) [0.46, 2.44]
		SoC	10	8 (80.0)	-5.75 (7.29)	-15.0	-7.50	4.0	
	Week 8	Peg	19	15 (78.9)	3.87 (9.20)	-12.0	5.00	25.0	0.95 (0.49) [0.09, 2.02]
		SoC	10	8 (80.0)	-4.38 (6.39)	-14.0	-4.50	6.0	
	Week 12	Peg	19	17 (89.5)	4.35 (8.51)	-9.0	5.00	23.0	1.00 (0.48) [0.16, 2.03]
		SoC	10	8 (80.0)	-3.63 (5.66)	-13.0	-3.00	5.0	
	Week 16	Peg	19	16 (84.2)	5.56 (8.83)	-8.0	6.00	24.0	1.13 (0.47) [0.31, 2.17]
		SoC	10	9 (90.0)	-3.56 (5.43)	-10.0	-4.00	6.0	
	Week 20	Peg	19	17 (89.5)	5.47 (10.17)	-13.0	7.00	25.0	0.92 (0.47) [0.08, 1.94]
		SoC	10	8 (80.0)	-3.00 (4.99)	-9.0	-3.50	5.0	
	Week 26	Peg	19	15 (78.9)	7.00 (9.76)	-8.0	7.00	25.0	0.79 (0.50) [-0.11, 1.88]
		SoC	10	7 (70.0)	-0.43 (6.95)	-8.0	-1.00	9.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SIHD: Change from baseline in FACIT Fatigue score (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in FACIT Fatigue score (censored)	Female	Week 4	Peg	16	15 (93.8)	9.47 (8.53)	-2.0	7.00	26.0	1.41 (0.54) [0.51, 2.64]
			SoC	8	7 (87.5)	-3.57 (9.71)	-20.0	-2.00	12.0	
		Week 8	Peg	16	14 (87.5)	11.00 (8.77)	1.0	8.00	28.0	1.28 (0.54) [0.37, 2.50]
			SoC	8	7 (87.5)	-1.29 (10.21)	-20.0	0.00	12.0	
		Week 12	Peg	16	14 (87.5)	12.14 (8.87)	0.0	9.00	28.0	1.36 (0.55) [0.45, 2.61]
			SoC	8	7 (87.5)	-1.14 (10.33)	-20.0	0.00	12.0	
		Week 16	Peg	16	14 (87.5)	11.43 (9.06)	0.0	9.50	28.0	1.19 (0.53) [0.28, 2.39]
			SoC	8	7 (87.5)	-0.14 (9.89)	-20.0	0.00	12.0	
		Week 20	Peg	16	15 (93.8)	11.40 (9.70)	1.0	8.00	29.0	1.24 (0.53) [0.34, 2.42]
			SoC	8	7 (87.5)	-1.29 (10.21)	-20.0	0.00	12.0	
		Week 26	Peg	16	13 (81.3)	12.38 (9.68)	-2.0	9.00	29.0	1.32 (0.58) [0.35, 2.66]
			SoC	8	6 (75.0)	-1.50 (10.80)	-20.0	0.50	12.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SICB: Change from baseline in FACIT Fatigue score (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.1232

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SICB: Change from baseline in FACIT Fatigue score (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Latin America	Peg	Week 4	12	11 (91.7)	13.61 (1.12)	(11.08, 16.15)	14.18 (2.88)	(7.67, 20.68)	0.0008	*
		Week 8	12	10 (83.3)	11.77 (1.72)	(7.80, 15.73)	12.60 (4.24)	(2.81, 22.38)	0.0179	*
		Week 12	12	11 (91.7)	12.90 (1.41)	(9.72, 16.08)	13.40 (3.61)	(5.23, 21.57)	0.0048	*
		Week 16	12	10 (83.3)	12.54 (1.32)	(9.50, 15.59)	13.75 (3.26)	(6.24, 21.26)	0.0029	*
		Week 20	12	10 (83.3)	14.84 (1.56)	(11.25, 18.44)	15.06 (3.90)	(6.06, 24.06)	0.0048	*
		Week 26	12	9 (75.0)	15.22 (1.77)	(11.04, 19.40)	15.21 (4.23)	(5.21, 25.21)	0.0088	*
	SoC	Week 4	2	2 (100.0)	-0.56 (2.64)	(-6.53, 5.41)				
		Week 8	2	2 (100.0)	-0.83 (3.87)	(-9.75, 8.09)				
		Week 12	2	2 (100.0)	-0.50 (3.31)	(-8.00, 6.99)				
		Week 16	2	2 (100.0)	-1.21 (2.97)	(-8.06, 5.64)				
		Week 20	2	2 (100.0)	-0.22 (3.55)	(-8.40, 7.96)				
		Week 26	2	2 (100.0)	0.01 (3.80)	(-8.98, 9.01)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SICB: Change from baseline in FACIT Fatigue score (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Asia Pacific Peg		Week 4	23	20 (87.0)	3.95 (1.51)	(0.86, 7.03)	8.87 (2.48)	(3.80, 13.94)	0.0012	*
		Week 8	23	19 (82.6)	4.63 (1.63)	(1.30, 7.96)	7.41 (2.63)	(2.02, 12.80)	0.0089	*
		Week 12	23	20 (87.0)	5.21 (1.55)	(2.04, 8.37)	7.96 (2.55)	(2.75, 13.17)	0.0040	*
		Week 16	23	20 (87.0)	5.90 (1.56)	(2.70, 9.09)	7.68 (2.48)	(2.61, 12.75)	0.0043	*
		Week 20	23	22 (95.7)	5.16 (1.65)	(1.79, 8.52)	7.46 (2.79)	(1.77, 13.15)	0.0118	*
		Week 26	23	19 (82.6)	6.17 (1.69)	(2.69, 9.64)	5.82 (2.90)	(-0.13, 11.77)	0.0550	
SoC		Week 4	16	13 (81.3)	-4.92 (1.91)	(-8.82, -1.03)				
		Week 8	16	13 (81.3)	-2.77 (1.99)	(-6.84, 1.29)				
		Week 12	16	13 (81.3)	-2.75 (1.96)	(-6.75, 1.25)				
		Week 16	16	14 (87.5)	-1.78 (1.88)	(-5.62, 2.06)				
		Week 20	16	13 (81.3)	-2.31 (2.19)	(-6.76, 2.15)				
		Week 26	16	11 (68.8)	0.35 (2.29)	(-4.36, 5.06)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SIHB: Change from baseline in FACIT Fatigue score (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
FACIT Fatigue score (censored)	Latin America	Baseline	Peg	12	12 (100.0)	31.67 (11.77)	12.0	31.00	50.0	
			SoC	2	2 (100.0)	37.50 (0.71)	37.0	37.50	38.0	
		Week 4	Peg	12	11 (91.7)	47.55 (3.17)	41.0	48.00	52.0	
			SoC	2	2 (100.0)	34.00 (7.07)	29.0	34.00	39.0	
		Week 8	Peg	12	10 (83.3)	46.70 (4.64)	36.0	47.50	52.0	
			SoC	2	2 (100.0)	34.00 (7.07)	29.0	34.00	39.0	
		Week 12	Peg	12	11 (91.7)	46.73 (4.56)	36.0	47.00	52.0	
			SoC	2	2 (100.0)	34.00 (7.07)	29.0	34.00	39.0	
		Week 16	Peg	12	10 (83.3)	47.40 (3.37)	43.0	48.50	52.0	
			SoC	2	2 (100.0)	34.00 (7.07)	29.0	34.00	39.0	
		Week 20	Peg	12	10 (83.3)	47.70 (4.83)	37.0	49.50	52.0	
			SoC	2	2 (100.0)	34.00 (7.07)	29.0	34.00	39.0	
		Week 26	Peg	12	9 (75.0)	47.56 (5.27)	36.0	50.00	52.0	
			SoC	2	2 (100.0)	34.00 (7.07)	29.0	34.00	39.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SIHB: Change from baseline in FACIT Fatigue score (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
FACIT Fatigue score (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	38.78 (9.40)	19.0	41.00	52.0	
			SoC	16	14 (87.5)	37.07 (10.01)	16.0	38.50	49.0	
		Week 4	Peg	23	20 (87.0)	42.55 (7.02)	29.0	44.50	52.0	
	SoC		16	15 (93.8)	33.60 (12.13)	7.0	33.00	48.0		
		Week 8	Peg	23	19 (82.6)	42.95 (8.89)	16.0	44.00	51.0	
	SoC		16	14 (87.5)	35.00 (9.88)	18.0	35.50	49.0		
		Week 12	Peg	23	20 (87.0)	43.85 (8.71)	20.0	47.00	52.0	
	SoC		16	14 (87.5)	35.50 (8.99)	21.0	34.00	49.0		
		Week 16	Peg	23	20 (87.0)	43.85 (8.40)	20.0	47.00	52.0	
	SoC		16	15 (93.8)	36.13 (9.33)	21.0	34.00	49.0		
		Week 20	Peg	23	22 (95.7)	43.27 (8.98)	15.0	46.00	52.0	
	SoC		16	15 (93.8)	36.27 (9.00)	21.0	38.00	49.0		
		Week 26	Peg	23	19 (82.6)	44.21 (7.93)	20.0	46.00	52.0	
	SoC		16	12 (75.0)	38.58 (8.90)	23.0	40.00	50.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SIHB: Change from baseline in FACIT Fatigue score (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in FACIT Fatigue score (censored)	Latin America	Week 4	Peg	12	11 (91.7)	14.09 (9.47)	-3.0	17.00	26.0	1.77 (0.97) [0.27, 4.07]
			SoC	2	2 (100.0)	-3.50 (6.36)	-8.0	-3.50	1.0	
		Week 8	Peg	12	10 (83.3)	12.30 (11.94)	-10.0	11.00	28.0	1.27 (0.93) [-0.26, 3.43]
			SoC	2	2 (100.0)	-3.50 (6.36)	-8.0	-3.50	1.0	
		Week 12	Peg	12	11 (91.7)	13.27 (10.18)	-4.0	12.00	28.0	1.58 (0.94) [0.07, 3.79]
			SoC	2	2 (100.0)	-3.50 (6.36)	-8.0	-3.50	1.0	
		Week 16	Peg	12	10 (83.3)	13.00 (9.93)	-2.0	12.00	28.0	1.58 (0.97) [0.06, 3.89]
			SoC	2	2 (100.0)	-3.50 (6.36)	-8.0	-3.50	1.0	
		Week 20	Peg	12	10 (83.3)	15.50 (8.55)	2.0	14.50	29.0	2.10 (1.04) [0.57, 4.66]
			SoC	2	2 (100.0)	-3.50 (6.36)	-8.0	-3.50	1.0	
		Week 26	Peg	12	9 (75.0)	15.89 (8.94)	2.0	14.00	29.0	2.04 (1.08) [0.50, 4.72]
			SoC	2	2 (100.0)	-3.50 (6.36)	-8.0	-3.50	1.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SIHB: Change from baseline in FACIT Fatigue score (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline in FACIT Fatigue score (censored)	Asia Pacific	Week 4	Peg	23	20 (87.0)	3.90 (6.02)	-8.0	3.00	16.0	1.20 (0.40)	[0.49, 2.08]
			SoC	16	13 (81.3)	-4.92 (8.71)	-20.0	-5.00	12.0		
	Week 8	Peg	23	19 (82.6)	4.68 (7.02)	-12.0	5.00	21.0	0.95 (0.39)	[0.24, 1.80]	
		SoC	16	13 (81.3)	-2.85 (8.69)	-20.0	-4.00	12.0			
	Week 12	Peg	23	20 (87.0)	4.90 (7.66)	-9.0	5.50	24.0	0.88 (0.39)	[0.19, 1.71]	
		SoC	16	13 (81.3)	-2.31 (8.39)	-20.0	-1.00	12.0			
	Week 16	Peg	23	20 (87.0)	5.95 (8.19)	-8.0	5.50	26.0	0.94 (0.38)	[0.26, 1.76]	
		SoC	16	14 (87.5)	-1.86 (7.96)	-20.0	-1.00	12.0			
	Week 20	Peg	23	22 (95.7)	4.95 (9.33)	-13.0	5.00	29.0	0.77 (0.37)	[0.08, 1.56]	
		SoC	16	13 (81.3)	-2.00 (7.99)	-20.0	-3.00	12.0			
	Week 26	Peg	23	19 (82.6)	6.47 (9.07)	-8.0	6.00	27.0	0.74 (0.41)	[0.00, 1.61]	
		SoC	16	11 (68.8)	-0.45 (9.06)	-20.0	0.00	12.0			

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SICA: Change from baseline in FACIT Fatigue score (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.7573

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SICA: Change from baseline in FACIT Fatigue score (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
< 4	Peg	Week 4	21	18 (85.7)	7.84 (1.45)	(4.81, 10.87)	12.47 (3.12)	(5.97, 18.97)	0.0007	*
		Week 8	21	17 (81.0)	7.36 (1.47)	(4.28, 10.44)	10.98 (3.09)	(4.52, 17.44)	0.0021	*
		Week 12	21	18 (85.7)	7.97 (1.37)	(5.10, 10.83)	12.64 (2.95)	(6.49, 18.79)	0.0004	*
		Week 16	21	17 (81.0)	9.43 (1.28)	(6.76, 12.11)	12.82 (2.52)	(7.57, 18.07)	<0.0001	*
		Week 20	21	18 (85.7)	8.86 (1.55)	(5.63, 12.09)	11.34 (3.33)	(4.39, 18.30)	0.0028	*
	Week 26	21	16 (76.2)	10.68 (1.49)	(7.54, 13.82)	9.65 (3.34)	(2.61, 16.68)	0.0101	*	
	SoC	Week 4	8	5 (62.5)	-4.63 (2.76)	(-10.38, 1.12)				
		Week 8	8	5 (62.5)	-3.62 (2.71)	(-9.30, 2.06)				
		Week 12	8	5 (62.5)	-4.68 (2.61)	(-10.11, 0.76)				
		Week 16	8	6 (75.0)	-3.39 (2.16)	(-7.90, 1.12)				
		Week 20	8	5 (62.5)	-2.49 (2.95)	(-8.63, 3.66)				
	Week 26	8	4 (50.0)	1.03 (2.98)	(-5.26, 7.32)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SICA: Change from baseline in FACIT Fatigue score (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	6.95 (2.17)	(2.43, 11.47)	11.59 (3.29)	(4.74, 18.45)	0.0021	*
		Week 8	14	12 (85.7)	7.11 (2.49)	(1.90, 12.32)	9.54 (3.69)	(1.81, 17.27)	0.0183	*
		Week 12	14	13 (92.9)	7.79 (2.16)	(3.28, 12.31)	9.22 (3.28)	(2.38, 16.07)	0.0108	*
		Week 16	14	13 (92.9)	6.62 (2.22)	(1.99, 11.25)	7.63 (3.37)	(0.61, 14.65)	0.0347	*
		Week 20	14	14 (100.0)	7.40 (2.27)	(2.68, 12.12)	9.36 (3.52)	(2.05, 16.68)	0.0146	*
		Week 26	14	12 (85.7)	7.19 (2.40)	(2.14, 12.24)	8.00 (3.68)	(0.27, 15.73)	0.0434	*
	SoC	Week 4	10	10 (100.0)	-4.64 (2.47)	(-9.79, 0.51)				
		Week 8	10	10 (100.0)	-2.43 (2.73)	(-8.14, 3.28)				
		Week 12	10	10 (100.0)	-1.43 (2.47)	(-6.58, 3.71)				
		Week 16	10	10 (100.0)	-1.01 (2.53)	(-6.29, 4.27)				
		Week 20	10	10 (100.0)	-1.96 (2.68)	(-7.54, 3.62)				
		Week 26	10	9 (90.0)	-0.81 (2.78)	(-6.64, 5.03)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SIHA: Change from baseline in FACIT Fatigue score (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
FACIT Fatigue score (censored)	< 4	Baseline	Peg	21	21 (100.0)	35.38 (11.27)	12.0	39.00	50.0	
			SoC	8	6 (75.0)	38.17 (9.79)	20.0	39.50	47.0	
	Week 4		Peg	21	18 (85.7)	44.83 (6.34)	29.0	46.00	52.0	
			SoC	8	7 (87.5)	36.14 (9.70)	24.0	33.00	48.0	
	Week 8		Peg	21	17 (81.0)	45.00 (6.94)	30.0	49.00	52.0	
			SoC	8	6 (75.0)	36.50 (9.03)	26.0	35.00	49.0	
	Week 12		Peg	21	18 (85.7)	44.94 (7.20)	24.0	48.00	52.0	
			SoC	8	6 (75.0)	35.33 (9.27)	25.0	32.50	49.0	
	Week 16		Peg	21	17 (81.0)	46.12 (6.23)	31.0	48.00	52.0	
			SoC	8	7 (87.5)	36.14 (8.84)	26.0	34.00	49.0	
	Week 20		Peg	21	18 (85.7)	44.50 (7.12)	26.0	46.50	52.0	
			SoC	8	7 (87.5)	37.43 (9.34)	25.0	38.00	49.0	
	Week 26		Peg	21	16 (76.2)	45.63 (5.97)	31.0	47.50	52.0	
			SoC	8	5 (62.5)	39.00 (10.51)	28.0	39.00	50.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SIHA: Change from baseline in FACIT Fatigue score (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
FACIT Fatigue score (censored)	>= 4	Baseline	Peg	14	14 (100.0)	37.79 (9.92)	19.0	41.00	52.0	
			SoC	10	10 (100.0)	36.50 (9.51)	16.0	38.00	49.0	
	Week 4	Peg	14	13 (92.9)	43.62 (6.59)	29.0	44.00	50.0		
		SoC	10	10 (100.0)	31.90 (12.78)	7.0	33.50	47.0		
	Week 8	Peg	14	12 (85.7)	43.17 (9.12)	16.0	44.50	51.0		
		SoC	10	10 (100.0)	33.90 (9.93)	18.0	36.50	45.0		
	Week 12	Peg	14	13 (92.9)	44.77 (8.30)	20.0	47.00	52.0		
		SoC	10	10 (100.0)	35.30 (8.65)	21.0	36.50	48.0		
	Week 16	Peg	14	13 (92.9)	43.62 (8.46)	20.0	45.00	52.0		
		SoC	10	10 (100.0)	35.70 (9.48)	21.0	36.50	48.0		
	Week 20	Peg	14	14 (100.0)	44.86 (9.50)	15.0	47.00	52.0		
		SoC	10	10 (100.0)	35.00 (8.47)	21.0	36.50	45.0		
	Week 26	Peg	14	12 (85.7)	44.83 (8.96)	20.0	47.00	52.0		
		SoC	10	9 (90.0)	37.33 (7.95)	23.0	39.00	50.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SIHA: Change from baseline in FACIT Fatigue score (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in FACIT Fatigue score (censored)	Week 4	Peg	21	18 (85.7)	7.94 (9.61)	-8.0	5.00	26.0	1.35 (0.58) [0.35, 2.63]
		SoC	8	5 (62.5)	-5.00 (7.68)	-14.0	-8.00	4.0	
	Week 8	Peg	21	17 (81.0)	7.35 (9.41)	-10.0	7.00	28.0	1.19 (0.58) [0.19, 2.46]
		SoC	8	5 (62.5)	-3.60 (6.35)	-10.0	-5.00	6.0	
	Week 12	Peg	21	18 (85.7)	8.06 (8.73)	-9.0	8.00	28.0	1.50 (0.59) [0.50, 2.82]
		SoC	8	5 (62.5)	-5.00 (6.82)	-13.0	-7.00	5.0	
	Week 16	Peg	21	17 (81.0)	9.71 (8.33)	-2.0	9.00	28.0	1.68 (0.58) [0.74, 3.00]
		SoC	8	6 (75.0)	-4.17 (6.52)	-10.0	-6.00	6.0	
	Week 20	Peg	21	18 (85.7)	9.17 (9.57)	-13.0	8.00	29.0	1.36 (0.58) [0.37, 2.65]
		SoC	8	5 (62.5)	-3.60 (6.23)	-9.0	-7.00	5.0	
	Week 26	Peg	21	16 (76.2)	10.88 (8.94)	-2.0	10.50	29.0	1.13 (0.63) [0.03, 2.51]
		SoC	8	4 (50.0)	0.25 (9.54)	-8.0	0.00	9.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWC_SIHA: Change from baseline in FACIT Fatigue score (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in FACIT Fatigue score (censored)	Week 4	Peg	14	13 (92.9)	6.92 (7.87)	-4.0	7.00	22.0	1.33 (0.50) [0.50, 2.45]
		SoC	10	10 (100.0)	-4.60 (8.93)	-20.0	-3.50	12.0	
	Week 8	Peg	14	12 (85.7)	7.25 (10.16)	-12.0	5.50	25.0	0.97 (0.48) [0.13, 2.03]
		SoC	10	10 (100.0)	-2.60 (9.32)	-20.0	-2.00	12.0	
	Week 12	Peg	14	13 (92.9)	7.62 (10.61)	-8.0	7.00	28.0	0.87 (0.47) [0.05, 1.89]
		SoC	10	10 (100.0)	-1.20 (8.52)	-20.0	-0.50	12.0	
	Week 16	Peg	14	13 (92.9)	6.46 (10.43)	-8.0	6.00	26.0	0.73 (0.46) [-0.09, 1.72]
		SoC	10	10 (100.0)	-0.80 (8.26)	-20.0	-0.50	12.0	
	Week 20	Peg	14	14 (100.0)	7.07 (11.30)	-13.0	8.00	29.0	0.81 (0.45) [-0.00, 1.79]
		SoC	10	10 (100.0)	-1.50 (8.44)	-20.0	-1.50	12.0	
	Week 26	Peg	14	12 (85.7)	7.67 (11.24)	-8.0	8.00	27.0	0.86 (0.49) [-0.01, 1.93]
		SoC	10	9 (90.0)	-1.44 (8.63)	-20.0	0.00	12.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1FAWIT8_SITC: Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by age
 ITT

Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWIT8_SITC: Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by age ITT

Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	19 (65.5)	4.21	(4.14, 12.14)	5.54	(1.24, 24.69)	0.0248	*
	SoC	13	3 (23.1)	26.29	(4.14, 26.29)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWIT8_SITC: Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by age
 ITT

Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	2 (33.3)	16.43	(4.57, NE)	NE	(NE, NE)	NE	
	SoC	5	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWIT8_SITD: Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by sex
 ITT

Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.4854

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWIT8_SITD: Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by sex
 ITT

Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	10 (52.6)	4.57	(4.14, 16.43)	10.38	(1.21, 89.07)	0.0328	*
	SoC	10	2 (20.0)	26.29	(26.14, 26.29)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWIT8_SITD: Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by sex
 ITT

Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	11 (68.8)	4.43	(4.14, 20.14)	3.55	(0.39, 32.40)	0.2609	
	SoC	8	1 (12.5)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWIT8_SITB: Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by region
 ITT

Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWIT8_SITB: Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by region
 ITT

Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	9 (75.0)	4.14	(4.14, 4.57)	NE	(NE, NE)	NE	
	SoC	2	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWIT8_SITB: Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by region
 ITT

Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	12 (52.2)	14.14	(4.14, 20.14)	3.95	(0.87, 17.94)	0.0752	
	SoC	16	3 (18.8)	26.29	(4.14, 26.29)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWIT8_SITA: Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by number of transfusions previous year
 ITT

Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								0.9338

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWIT8_SITA: Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by number of transfusions previous year
 ITT

Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	13 (61.9)	4.57	(4.14, 12.14)	7.32	(0.93, 57.79)	0.0591	
	SoC	8	2 (25.0)	26.21	(26.14, 26.29)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWIT8_SITA: Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by number of transfusions previous year
 ITT

Time to increase of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	8 (57.1)	4.21	(4.14, 20.14)	6.87	(0.84, 56.02)	0.0717	
	SoC	10	1 (10.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWDT8_SITC: Time to decrease of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by age
 ITT

Time to decrease of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWDT8_SITC: Time to decrease of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by age
 ITT

Time to decrease of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	4 (13.8)	NE	(NE, NE)	0.16	(0.04, 0.60)	0.0065	*
	SoC	13	6 (46.2)	16.43	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWDT8_SITC: Time to decrease of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by age
 ITT

Time to decrease of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	2 (33.3)	NE	(12.29, NE)	NE	(NE, NE)	NE	
	SoC	5	3 (60.0)	4.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWDT8_SITD: Time to decrease of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by sex
 ITT

Time to decrease of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWDT8_SITD: Time to decrease of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by sex
 ITT

Time to decrease of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	6 (31.6)	NE	(20.43, NE)	0.24	(0.07, 0.80)	0.0198	*
	SoC	10	6 (60.0)	8.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWDT8_SITD: Time to decrease of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by sex
 ITT

Time to decrease of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	0 (0.0)	NE	(NE, NE)	NE	(NE, NE)	NE	
	SoC	8	3 (37.5)	12.29	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWDT8_SITB: Time to decrease of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by region
 ITT

Time to decrease of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWDT8_SITB: Time to decrease of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by region
 ITT

Time to decrease of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	1 (8.3)	NE	(NE, NE)	NE	(NE, NE)	NE	
	SoC	2	1 (50.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1FAWDT8_SITB: Time to decrease of at least 8 points in change from baseline of FACIT-fatigue-score (censored) by region
 ITT

Time to decrease of at least 8 points in change from baseline of FACIT-fatigue-score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	5 (21.7)	NE	(NE, NE)	0.20	(0.06, 0.66)	0.0076	*
	SoC	16	8 (50.0)	12.29	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWC_SICC: Change from baseline in QLQ-C30 Pain (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.6604

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SICC: Change from baseline in QLQ-C30 Pain (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 60	Peg	Week 4	29	26 (89.7)	1.72 (3.85)	(-6.10, 9.55)	-0.57 (7.22)	(-15.25, 14.11)	0.9375	
		Week 8	29	24 (82.8)	-1.17 (3.86)	(-9.05, 6.70)	-0.89 (7.05)	(-15.27, 13.48)	0.8998	
		Week 12	29	26 (89.7)	-2.66 (3.84)	(-10.48, 5.16)	-5.64 (7.21)	(-20.31, 9.02)	0.4393	
		Week 16	29	25 (86.2)	-0.81 (4.11)	(-9.17, 7.54)	0.13 (7.36)	(-14.84, 15.10)	0.9857	
		Week 20	29	27 (93.1)	-2.01 (4.01)	(-10.16, 6.15)	-2.39 (7.64)	(-17.91, 13.12)	0.7558	
		Week 26	29	23 (79.3)	-0.76 (4.10)	(-9.14, 7.63)	2.83 (7.79)	(-13.11, 18.77)	0.7189	
	SoC	Week 4	13	11 (84.6)	2.29 (5.97)	(-9.85, 14.43)				
		Week 8	13	11 (84.6)	-0.28 (5.75)	(-12.00, 11.45)				
		Week 12	13	11 (84.6)	2.98 (5.96)	(-9.14, 15.11)				
		Week 16	13	12 (92.3)	-0.95 (5.97)	(-13.09, 11.20)				
		Week 20	13	11 (84.6)	0.39 (6.38)	(-12.57, 13.34)				
		Week 26	13	10 (76.9)	-3.59 (6.42)	(-16.72, 9.54)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SICC: Change from baseline in QLQ-C30 Pain (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	1.11 (7.87)	(-19.13, 21.34)	18.27 (13.19)	(-15.64, 52.19)	0.2246	
		Week 8	6	5 (83.3)	-0.95 (7.07)	(-19.14, 17.23)	-1.32 (11.86)	(-31.79, 29.16)	0.9159	
		Week 12	6	5 (83.3)	-3.70 (12.39)	(-35.53, 28.14)	-15.64 (20.76)	(-69.00, 37.72)	0.4850	
		Week 16	6	5 (83.3)	-0.12 (13.15)	(-33.91, 33.68)	-17.41 (21.96)	(-73.85, 39.03)	0.4637	
		Week 20	6	5 (83.3)	2.72 (13.68)	(-32.44, 37.88)	-10.79 (22.84)	(-69.51, 47.93)	0.6566	
	Week 26	6	4 (66.7)	12.78 (5.29)	(-9.96, 35.52)	23.33 (10.80)	(-23.14, 69.81)	0.1633		
	SoC	Week 4	5	4 (80.0)	-17.17 (9.39)	(-41.31, 6.98)				
		Week 8	5	4 (80.0)	0.36 (8.44)	(-21.33, 22.06)				
		Week 12	5	4 (80.0)	11.95 (14.78)	(-26.04, 49.93)				
		Week 16	5	4 (80.0)	17.30 (15.72)	(-23.11, 57.70)				
		Week 20	5	4 (80.0)	13.51 (16.35)	(-28.53, 55.55)				
	Week 26	5	2 (40.0)	-10.56 (9.53)	(-51.58, 30.47)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SIHC: Change from baseline in QLQ-C30 Pain (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Pain (censored)	< 60	Baseline	Peg	29	29 (100.0)	16.09 (24.59)	0.0	0.00	100.0	
			SoC	13	12 (92.3)	20.83 (22.61)	0.0	16.67	66.7	
		Week 4	Peg	29	26 (89.7)	16.03 (20.81)	0.0	8.33	83.3	
			SoC	13	12 (92.3)	18.06 (16.60)	0.0	16.67	50.0	
		Week 8	Peg	29	24 (82.8)	13.19 (19.65)	0.0	0.00	66.7	
			SoC	13	11 (84.6)	16.67 (16.67)	0.0	16.67	50.0	
		Week 12	Peg	29	26 (89.7)	12.82 (20.17)	0.0	0.00	66.7	
			SoC	13	11 (84.6)	18.18 (17.41)	0.0	16.67	50.0	
		Week 16	Peg	29	25 (86.2)	14.67 (21.69)	0.0	0.00	83.3	
			SoC	13	12 (92.3)	13.89 (15.62)	0.0	8.33	33.3	
		Week 20	Peg	29	27 (93.1)	12.35 (23.38)	0.0	0.00	66.7	
			SoC	13	12 (92.3)	16.67 (15.89)	0.0	16.67	33.3	
		Week 26	Peg	29	23 (79.3)	14.49 (20.90)	0.0	0.00	66.7	
			SoC	13	10 (76.9)	15.00 (14.59)	0.0	16.67	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SIHC: Change from baseline in QLQ-C30 Pain (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Pain (censored)	>= 60	Baseline	Peg	6	6 (100.0)	11.11 (20.18)	0.0	0.00	50.0	
			SoC	5	4 (80.0)	33.33 (36.00)	0.0	25.00	83.3	
		Week 4	Peg	6	5 (83.3)	10.00 (14.91)	0.0	0.00	33.3	
			SoC	5	5 (100.0)	16.67 (37.27)	0.0	0.00	83.3	
		Week 8	Peg	6	5 (83.3)	13.33 (18.26)	0.0	0.00	33.3	
			SoC	5	5 (100.0)	23.33 (22.36)	0.0	33.33	50.0	
		Week 12	Peg	6	5 (83.3)	13.33 (18.26)	0.0	0.00	33.3	
			SoC	5	5 (100.0)	30.00 (29.81)	0.0	33.33	66.7	
		Week 16	Peg	6	5 (83.3)	20.00 (13.94)	0.0	16.67	33.3	
			SoC	5	5 (100.0)	33.33 (35.36)	0.0	33.33	83.3	
		Week 20	Peg	6	5 (83.3)	23.33 (22.36)	0.0	33.33	50.0	
			SoC	5	5 (100.0)	30.00 (29.81)	0.0	33.33	66.7	
		Week 26	Peg	6	4 (66.7)	25.00 (16.67)	0.0	33.33	33.3	
			SoC	5	3 (60.0)	11.11 (19.25)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SIHC: Change from baseline in QLQ-C30 Pain (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline < 60 in QLQ-C30 Pain (censored)	Week 4	Peg	29	26 (89.7)	2.56 (26.54)	-33.3	0.00	83.3	0.10	(0.37) [-0.62, 0.84]
		SoC	13	11 (84.6)	0.00 (19.72)	-33.3	0.00	33.3		
	Week 8	Peg	29	24 (82.8)	0.00 (24.08)	-50.0	0.00	66.7	0.12	(0.38) [-0.61, 0.87]
		SoC	13	11 (84.6)	-3.03 (24.52)	-33.3	0.00	33.3		
	Week 12	Peg	29	26 (89.7)	-0.64 (26.03)	-66.7	0.00	66.7	0.03	(0.37) [-0.69, 0.77]
		SoC	13	11 (84.6)	-1.52 (22.92)	-50.0	0.00	33.3		
	Week 16	Peg	29	25 (86.2)	2.00 (32.74)	-66.7	0.00	83.3	0.28	(0.36) [-0.41, 1.02]
		SoC	13	12 (92.3)	-6.94 (26.07)	-50.0	0.00	33.3		
	Week 20	Peg	29	27 (93.1)	-1.23 (24.86)	-50.0	0.00	66.7	0.01	(0.37) [-0.71, 0.74]
		SoC	13	11 (84.6)	-1.52 (21.67)	-33.3	0.00	33.3		
	Week 26	Peg	29	23 (79.3)	0.72 (24.35)	-33.3	0.00	66.7	0.28	(0.39) [-0.48, 1.07]
		SoC	13	10 (76.9)	-6.67 (29.61)	-50.0	0.00	33.3		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SIHC: Change from baseline in QLQ-C30 Pain (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in QLQ-C30 Pain (censored)	Week 4	Peg	6	5 (83.3)	0.00 (23.57)	-33.3	0.00	33.3	0.54 (0.82) [-0.88, 2.37]
		SoC	5	4 (80.0)	-12.50 (15.96)	-33.3	-8.33	0.0	
	Week 8	Peg	6	5 (83.3)	3.33 (18.26)	-16.7	0.00	33.3	0.34 (0.80) [-1.13, 2.08]
		SoC	5	4 (80.0)	-4.17 (20.97)	-33.3	0.00	16.7	
	Week 12	Peg	6	5 (83.3)	3.33 (34.16)	-50.0	0.00	33.3	-0.02 (0.79) [-1.62, 1.55]
		SoC	5	4 (80.0)	4.17 (28.46)	-33.3	8.33	33.3	
	Week 16	Peg	6	5 (83.3)	6.67 (27.89)	-33.3	0.00	33.3	-0.05 (0.79) [-1.65, 1.52]
		SoC	5	4 (80.0)	8.33 (34.69)	-33.3	8.33	50.0	
	Week 20	Peg	6	5 (83.3)	10.00 (36.51)	-50.0	33.33	33.3	0.16 (0.80) [-1.37, 1.81]
		SoC	5	4 (80.0)	4.17 (28.46)	-33.3	8.33	33.3	
	Week 26	Peg	6	4 (66.7)	12.50 (25.00)	-16.7	16.67	33.3	0.15 (1.23) [-2.14, 2.68]
		SoC	5	2 (40.0)	8.33 (11.79)	0.0	8.33	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SICD: Change from baseline in QLQ-C30 Pain (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.1766

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SICD: Change from baseline in QLQ-C30 Pain (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Male	Peg	Week 4	19	16 (84.2)	3.62 (5.96)	(-8.82, 16.05)	3.26 (10.45)	(-18.55, 25.07)	0.7586	
		Week 8	19	15 (78.9)	8.76 (5.58)	(-2.92, 20.44)	7.40 (9.72)	(-12.95, 27.75)	0.4561	
		Week 12	19	17 (89.5)	11.59 (5.11)	(0.96, 22.22)	5.17 (9.26)	(-14.08, 24.42)	0.5826	
		Week 16	19	16 (84.2)	7.77 (6.80)	(-6.37, 21.91)	1.89 (11.74)	(-22.53, 26.30)	0.8739	
		Week 20	19	17 (89.5)	7.41 (6.20)	(-5.49, 20.31)	3.69 (11.13)	(-19.45, 26.84)	0.7432	
		Week 26	19	14 (73.7)	8.66 (5.77)	(-3.58, 20.89)	12.19 (11.06)	(-11.26, 35.64)	0.2867	
	SoC	Week 4	10	8 (80.0)	0.36 (8.38)	(-17.12, 17.83)				
		Week 8	10	8 (80.0)	1.36 (7.68)	(-14.70, 17.43)				
		Week 12	10	8 (80.0)	6.42 (7.50)	(-9.17, 22.01)				
		Week 16	10	9 (90.0)	5.89 (9.18)	(-13.20, 24.97)				
		Week 20	10	8 (80.0)	3.72 (9.05)	(-15.10, 22.53)				
		Week 26	10	6 (60.0)	-3.53 (9.07)	(-22.76, 15.69)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SICD: Change from baseline in QLQ-C30 Pain (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Female	Peg	Week 4	16	15 (93.8)	-2.15 (4.19)	(-10.96, 6.65)	1.96 (8.87)	(-16.68, 20.60)	0.8275	
		Week 8	16	14 (87.5)	-11.64 (3.02)	(-18.01, -5.26)	-10.13 (6.35)	(-23.52, 3.25)	0.1287	
		Week 12	16	14 (87.5)	-18.82 (3.30)	(-25.78, -11.85)	-20.89 (6.94)	(-35.52, -6.25)	0.0079	*
		Week 16	16	14 (87.5)	-10.92 (4.58)	(-20.58, -1.27)	-14.38 (9.61)	(-34.66, 5.89)	0.1527	
		Week 20	16	15 (93.8)	-11.46 (3.43)	(-18.66, -4.26)	-11.67 (7.26)	(-26.92, 3.58)	0.1253	
	Week 26	16	13 (81.3)	-6.59 (4.17)	(-15.47, 2.29)	-5.29 (8.90)	(-24.26, 13.68)	0.5609		
	SoC	Week 4	8	7 (87.5)	-4.12 (7.33)	(-19.51, 11.28)				
		Week 8	8	7 (87.5)	-1.51 (5.09)	(-12.23, 9.22)				
		Week 12	8	7 (87.5)	2.07 (5.56)	(-9.66, 13.80)				
		Week 16	8	7 (87.5)	3.46 (7.70)	(-12.79, 19.71)				
		Week 20	8	7 (87.5)	0.21 (6.00)	(-12.39, 12.81)				
Week 26	8	6 (75.0)	-1.30 (7.31)	(-16.89, 14.29)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SIHD: Change from baseline in QLQ-C30 Pain (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Pain (censored)	Male	Baseline	Peg	19	19 (100.0)	10.53 (16.86)	0.0	0.00	50.0	
			SoC	10	9 (90.0)	24.07 (22.22)	0.0	33.33	66.7	
		Week 4	Peg	19	16 (84.2)	16.67 (24.34)	0.0	0.00	83.3	
			SoC	10	9 (90.0)	14.81 (17.57)	0.0	16.67	50.0	
		Week 8	Peg	19	15 (78.9)	20.00 (22.89)	0.0	16.67	66.7	
			SoC	10	9 (90.0)	16.67 (18.63)	0.0	16.67	50.0	
		Week 12	Peg	19	17 (89.5)	23.53 (21.29)	0.0	16.67	66.7	
			SoC	10	9 (90.0)	22.22 (23.57)	0.0	16.67	66.7	
		Week 16	Peg	19	16 (84.2)	21.88 (24.13)	0.0	16.67	83.3	
			SoC	10	10 (100.0)	18.33 (26.59)	0.0	8.33	83.3	
		Week 20	Peg	19	17 (89.5)	22.55 (24.96)	0.0	16.67	66.7	
			SoC	10	9 (90.0)	18.52 (22.74)	0.0	16.67	66.7	
		Week 26	Peg	19	14 (73.7)	23.81 (22.37)	0.0	25.00	66.7	
			SoC	10	7 (70.0)	11.90 (12.60)	0.0	16.67	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SIHD: Change from baseline in QLQ-C30 Pain (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Pain (censored)	Female	Baseline	Peg	16	16 (100.0)	20.83 (29.50)	0.0	8.33	100.0	
			SoC	8	7 (87.5)	23.81 (31.71)	0.0	16.67	83.3	
		Week 4	Peg	16	15 (93.8)	13.33 (14.36)	0.0	16.67	33.3	
			SoC	8	8 (100.0)	20.83 (29.21)	0.0	8.33	83.3	
		Week 8	Peg	16	14 (87.5)	5.95 (10.56)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	21.43 (18.54)	0.0	16.67	50.0	
		Week 12	Peg	16	14 (87.5)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	8	7 (87.5)	21.43 (20.89)	0.0	33.33	50.0	
		Week 16	Peg	16	14 (87.5)	8.33 (12.66)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	21.43 (20.89)	0.0	33.33	50.0	
		Week 20	Peg	16	15 (93.8)	4.44 (17.21)	0.0	0.00	66.7	
			SoC	8	8 (100.0)	22.92 (19.80)	0.0	33.33	50.0	
		Week 26	Peg	16	13 (81.3)	7.69 (14.62)	0.0	0.00	33.3	
			SoC	8	6 (75.0)	16.67 (18.26)	0.0	16.67	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SIHD: Change from baseline in QLQ-C30 Pain (censored) by sex
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Male in QLQ-C30 Pain (censored)	Week 4	Peg	19	16 (84.2)	6.25 (30.35)	-33.3	0.00	83.3	0.45 (0.46) [-0.41, 1.40]
		SoC	10	8 (80.0)	-6.25 (17.68)	-33.3	-8.33	16.7	
	Week 8	Peg	19	15 (78.9)	11.11 (23.29)	-16.7	0.00	66.7	0.63 (0.47) [-0.23, 1.64]
		SoC	10	8 (80.0)	-4.17 (23.15)	-33.3	0.00	33.3	
	Week 12	Peg	19	17 (89.5)	13.73 (23.00)	-33.3	16.67	66.7	0.52 (0.46) [-0.33, 1.47]
		SoC	10	8 (80.0)	2.08 (18.77)	-16.7	0.00	33.3	
	Week 16	Peg	19	16 (84.2)	12.50 (33.05)	-50.0	0.00	83.3	0.50 (0.44) [-0.33, 1.42]
		SoC	10	9 (90.0)	-3.70 (28.60)	-33.3	0.00	50.0	
	Week 20	Peg	19	17 (89.5)	10.78 (30.01)	-50.0	0.00	66.7	0.44 (0.45) [-0.41, 1.38]
		SoC	10	8 (80.0)	-2.08 (24.30)	-33.3	0.00	33.3	
	Week 26	Peg	19	14 (73.7)	11.90 (28.06)	-33.3	0.00	66.7	0.79 (0.54) [-0.17, 1.96]
		SoC	10	6 (60.0)	-11.11 (27.22)	-50.0	-8.33	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SIHD: Change from baseline in QLQ-C30 Pain (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Pain (censored)	Female	Week 4	Peg	16	15 (93.8)	-2.22 (19.79)	-33.3	0.00	33.3	-0.11 (0.48) [-1.07, 0.84]
			SoC	8	7 (87.5)	0.00 (21.52)	-33.3	0.00	33.3	
		Week 8	Peg	16	14 (87.5)	-10.71 (16.80)	-50.0	0.00	0.0	-0.41 (0.49) [-1.44, 0.52]
			SoC	8	7 (87.5)	-2.38 (24.40)	-33.3	0.00	33.3	
		Week 12	Peg	16	14 (87.5)	-16.67 (21.68)	-66.7	-8.33	0.0	-0.56 (0.50) [-1.62, 0.36]
			SoC	8	7 (87.5)	-2.38 (29.55)	-50.0	0.00	33.3	
		Week 16	Peg	16	14 (87.5)	-8.33 (26.75)	-66.7	-8.33	33.3	-0.21 (0.49) [-1.20, 0.74]
			SoC	8	7 (87.5)	-2.38 (29.55)	-50.0	0.00	33.3	
		Week 20	Peg	16	15 (93.8)	-11.11 (16.27)	-50.0	0.00	0.0	-0.71 (0.50) [-1.77, 0.19]
			SoC	8	7 (87.5)	2.38 (22.42)	-33.3	0.00	33.3	
		Week 26	Peg	16	13 (81.3)	-7.69 (14.62)	-33.3	0.00	16.7	-0.50 (0.53) [-1.63, 0.48]
			SoC	8	6 (75.0)	2.78 (28.71)	-50.0	8.33	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SICB: Change from baseline in QLQ-C30 Pain (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.7480

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SICB: Change from baseline in QLQ-C30 Pain (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Latin America	Peg	Week 4	12	11 (91.7)	-11.40 (1.87)	(-15.64, -7.17)	-9.81 (4.76)	(-20.58, 0.96)	0.0693	
		Week 8	12	10 (83.3)	-6.47 (3.32)	(-14.13, 1.18)	-5.51 (8.13)	(-24.27, 13.24)	0.5168	
		Week 12	12	11 (91.7)	-5.65 (4.46)	(-15.74, 4.43)	-2.67 (11.34)	(-28.33, 22.98)	0.8190	
		Week 16	12	10 (83.3)	-6.35 (4.09)	(-15.79, 3.10)	-4.75 (10.03)	(-27.88, 18.39)	0.6487	
		Week 20	12	10 (83.3)	-11.39 (4.98)	(-22.88, 0.10)	-10.01 (12.24)	(-38.24, 18.23)	0.4374	
		Week 26	12	9 (75.0)	-4.53 (5.75)	(-18.13, 9.08)	-1.00 (13.52)	(-32.97, 30.97)	0.9431	
	SoC	Week 4	2	2 (100.0)	-1.59 (4.38)	(-11.49, 8.31)				
		Week 8	2	2 (100.0)	-0.96 (7.42)	(-18.08, 16.16)				
		Week 12	2	2 (100.0)	-2.98 (10.43)	(-26.57, 20.61)				
		Week 16	2	2 (100.0)	-1.60 (9.16)	(-22.72, 19.52)				
		Week 20	2	2 (100.0)	-1.38 (11.17)	(-27.14, 24.37)				
		Week 26	2	2 (100.0)	-3.53 (12.22)	(-32.43, 25.38)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SICB: Change from baseline in QLQ-C30 Pain (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Asia Pacific Peg		Week 4	23	20 (87.0)	8.58 (4.96)	(-1.57, 18.72)	10.71 (8.52)	(-6.71, 28.14)	0.2186	
		Week 8	23	19 (82.6)	1.98 (4.76)	(-7.76, 11.73)	2.32 (8.02)	(-14.10, 18.74)	0.7742	
		Week 12	23	20 (87.0)	-0.51 (5.00)	(-10.73, 9.71)	-6.56 (8.58)	(-24.11, 10.99)	0.4507	
		Week 16	23	20 (87.0)	2.76 (5.82)	(-9.12, 14.64)	-0.44 (9.66)	(-20.16, 19.28)	0.9639	
		Week 20	23	22 (95.7)	2.61 (5.11)	(-7.82, 13.04)	-3.16 (9.06)	(-21.63, 15.31)	0.7296	
		Week 26	23	18 (78.3)	3.43 (4.84)	(-6.55, 13.41)	5.26 (9.14)	(-13.61, 24.13)	0.5704	
SoC		Week 4	16	13 (81.3)	-2.14 (6.38)	(-15.18, 10.90)				
		Week 8	16	13 (81.3)	-0.34 (5.89)	(-12.40, 11.73)				
		Week 12	16	13 (81.3)	6.05 (6.42)	(-7.08, 19.19)				
		Week 16	16	14 (87.5)	3.20 (7.09)	(-11.29, 17.69)				
		Week 20	16	13 (81.3)	5.77 (6.93)	(-8.35, 19.90)				
		Week 26	16	10 (62.5)	-1.83 (7.22)	(-16.73, 13.07)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SIHB: Change from baseline in QLQ-C30 Pain (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Pain (censored)	Latin America	Baseline	Peg	12	12 (100.0)	26.39 (33.68)	0.0	8.33	100.0	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 4	Peg	12	11 (91.7)	7.58 (11.46)	0.0	0.00	33.3	
	SoC		2	2 (100.0)	16.67 (0.00)	16.7	16.67	16.7		
		Week 8	Peg	12	10 (83.3)	11.67 (13.72)	0.0	8.33	33.3	
	SoC		2	2 (100.0)	16.67 (0.00)	16.7	16.67	16.7		
		Week 12	Peg	12	11 (91.7)	13.64 (14.56)	0.0	16.67	33.3	
	SoC		2	2 (100.0)	16.67 (0.00)	16.7	16.67	16.7		
		Week 16	Peg	12	10 (83.3)	11.67 (13.72)	0.0	8.33	33.3	
	SoC		2	2 (100.0)	16.67 (0.00)	16.7	16.67	16.7		
		Week 20	Peg	12	10 (83.3)	10.00 (22.50)	0.0	0.00	66.7	
	SoC		2	2 (100.0)	16.67 (0.00)	16.7	16.67	16.7		
		Week 26	Peg	12	9 (75.0)	18.52 (19.44)	0.0	16.67	50.0	
	SoC		2	2 (100.0)	16.67 (0.00)	16.7	16.67	16.7		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SIHB: Change from baseline in QLQ-C30 Pain (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Pain (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	9.42 (14.06)	0.0	0.00	50.0	
			SoC	16	14 (87.5)	25.00 (26.75)	0.0	16.67	83.3	
		Week 4	Peg	23	20 (87.0)	19.17 (22.47)	0.0	16.67	83.3	
			SoC	16	15 (93.8)	17.78 (24.77)	0.0	0.00	83.3	
		Week 8	Peg	23	19 (82.6)	14.04 (21.70)	0.0	0.00	66.7	
			SoC	16	14 (87.5)	19.05 (19.46)	0.0	16.67	50.0	
		Week 12	Peg	23	20 (87.0)	12.50 (22.21)	0.0	0.00	66.7	
			SoC	16	14 (87.5)	22.62 (23.21)	0.0	25.00	66.7	
		Week 16	Peg	23	20 (87.0)	17.50 (23.24)	0.0	16.67	83.3	
			SoC	16	15 (93.8)	20.00 (25.35)	0.0	0.00	83.3	
		Week 20	Peg	23	22 (95.7)	15.91 (23.84)	0.0	0.00	66.7	
			SoC	16	15 (93.8)	21.11 (22.24)	0.0	33.33	66.7	
		Week 26	Peg	23	18 (78.3)	14.81 (21.30)	0.0	0.00	66.7	
			SoC	16	11 (68.8)	13.64 (16.36)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SIHb: Change from baseline in QLQ-C30 Pain (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Pain (censored)	Latin America	Week 4	Peg	12	11 (91.7)	-12.12 (18.40)	-33.3	0.00	16.7	-0.60 (0.86) [-2.45, 0.99]
			SoC	2	2 (100.0)	0.00 (23.57)	-16.7	0.00	16.7	
		Week 8	Peg	12	10 (83.3)	-6.67 (19.56)	-50.0	0.00	16.7	-0.31 (0.87) [-2.12, 1.35]
			SoC	2	2 (100.0)	0.00 (23.57)	-16.7	0.00	16.7	
		Week 12	Peg	12	11 (91.7)	-6.06 (32.72)	-66.7	0.00	33.3	-0.18 (0.85) [-1.91, 1.48]
			SoC	2	2 (100.0)	0.00 (23.57)	-16.7	0.00	16.7	
		Week 16	Peg	12	10 (83.3)	-6.67 (34.43)	-66.7	0.00	33.3	-0.18 (0.87) [-1.96, 1.50]
			SoC	2	2 (100.0)	0.00 (23.57)	-16.7	0.00	16.7	
		Week 20	Peg	12	10 (83.3)	-11.67 (20.86)	-50.0	0.00	0.0	-0.51 (0.88) [-2.38, 1.11]
			SoC	2	2 (100.0)	0.00 (23.57)	-16.7	0.00	16.7	
		Week 26	Peg	12	9 (75.0)	-5.56 (22.05)	-33.3	0.00	33.3	-0.23 (0.89) [-2.07, 1.48]
			SoC	2	2 (100.0)	0.00 (23.57)	-16.7	0.00	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SIHb: Change from baseline in QLQ-C30 Pain (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline in QLQ-C30 Pain (censored)	Asia Pacific	Week 4	Peg	23	20 (87.0)	10.00 (26.16)	-33.3	0.00	83.3	0.57 (0.38)	[-0.13, 1.35]
			SoC	16	13 (81.3)	-3.85 (19.43)	-33.3	0.00	33.3		
		Week 8	Peg	23	19 (82.6)	4.39 (24.12)	-33.3	0.00	66.7	0.33 (0.38)	[-0.38, 1.10]
			SoC	16	13 (81.3)	-3.85 (23.72)	-33.3	0.00	33.3		
		Week 12	Peg	23	20 (87.0)	3.33 (23.32)	-33.3	0.00	66.7	0.14 (0.37)	[-0.58, 0.87]
			SoC	16	13 (81.3)	0.00 (24.53)	-50.0	0.00	33.3		
		Week 16	Peg	23	20 (87.0)	7.50 (29.85)	-33.3	0.00	83.3	0.36 (0.36)	[-0.33, 1.11]
			SoC	16	14 (87.5)	-3.57 (29.37)	-50.0	0.00	50.0		
		Week 20	Peg	23	22 (95.7)	6.06 (27.48)	-33.3	0.00	66.7	0.23 (0.36)	[-0.47, 0.96]
			SoC	16	13 (81.3)	0.00 (23.57)	-33.3	0.00	33.3		
		Week 26	Peg	23	18 (78.3)	6.48 (25.01)	-33.3	0.00	66.7	0.42 (0.42)	[-0.36, 1.28]
			SoC	16	10 (62.5)	-5.00 (29.45)	-50.0	0.00	33.3		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SICA: Change from baseline in QLQ-C30 Pain (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.6766

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SICA: Change from baseline in QLQ-C30 Pain (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 4	21	18 (85.7)	-6.28 (3.79)	(-14.19, 1.63)	1.10 (8.53)	(-16.71, 18.90)	0.8991	
		Week 8	21	17 (81.0)	-4.12 (3.94)	(-12.38, 4.13)	5.19 (8.73)	(-13.08, 23.45)	0.5594	
		Week 12	21	18 (85.7)	-4.19 (5.51)	(-15.69, 7.32)	-9.26 (12.41)	(-35.14, 16.62)	0.4642	
		Week 16	21	17 (81.0)	-7.28 (5.56)	(-18.88, 4.31)	-8.47 (11.49)	(-32.43, 15.49)	0.4694	
		Week 20	21	18 (85.7)	-6.55 (5.68)	(-18.41, 5.30)	-6.82 (12.70)	(-33.31, 19.68)	0.5974	
	Week 26	21	15 (71.4)	-5.96 (4.32)	(-15.17, 3.25)	14.23 (11.71)	(-10.73, 39.20)	0.2431		
	SoC	Week 4	8	5 (62.5)	-7.38 (7.45)	(-22.93, 8.17)				
		Week 8	8	5 (62.5)	-9.31 (7.55)	(-25.12, 6.50)				
		Week 12	8	5 (62.5)	5.07 (10.84)	(-17.53, 27.67)				
		Week 16	8	6 (75.0)	1.19 (9.69)	(-19.03, 21.41)				
		Week 20	8	5 (62.5)	0.26 (11.11)	(-22.91, 23.44)				
Week 26		8	3 (37.5)	-20.20 (10.50)	(-42.57, 2.18)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SICA: Change from baseline in QLQ-C30 Pain (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	8.28 (6.36)	(-5.00, 21.55)	2.37 (9.77)	(-18.01, 22.75)	0.8105	
		Week 8	14	12 (85.7)	1.22 (5.60)	(-10.50, 12.94)	-5.64 (8.39)	(-23.20, 11.91)	0.5091	
		Week 12	14	13 (92.9)	-1.21 (4.68)	(-10.98, 8.57)	-7.77 (7.19)	(-22.77, 7.23)	0.2926	
		Week 16	14	13 (92.9)	6.26 (6.37)	(-7.03, 19.55)	-0.60 (9.78)	(-21.00, 19.80)	0.9516	
		Week 20	14	14 (100.0)	4.01 (5.25)	(-6.91, 14.93)	-3.70 (8.25)	(-20.87, 13.47)	0.6585	
		Week 26	14	12 (85.7)	9.17 (5.95)	(-3.33, 21.68)	2.89 (9.09)	(-16.22, 22.00)	0.7544	
	SoC	Week 4	10	10 (100.0)	5.90 (7.28)	(-9.27, 21.08)				
		Week 8	10	10 (100.0)	6.87 (6.14)	(-5.99, 19.73)				
		Week 12	10	10 (100.0)	6.57 (5.36)	(-4.61, 17.74)				
		Week 16	10	10 (100.0)	6.86 (7.29)	(-8.34, 22.06)				
		Week 20	10	10 (100.0)	7.71 (6.24)	(-5.26, 20.69)				
		Week 26	10	9 (90.0)	6.29 (6.87)	(-8.15, 20.72)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SIHA: Change from baseline in QLQ-C30 Pain (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Pain (censored)	< 4	Baseline	Peg	21	21 (100.0)	19.84 (26.15)	0.0	16.67	100.0	
			SoC	8	6 (75.0)	33.33 (21.08)	0.0	33.33	66.7	
	Week 4	Peg	21	18 (85.7)	12.04 (15.97)	0.0	0.00	50.0		
		SoC	8	7 (87.5)	11.90 (18.54)	0.0	0.00	50.0		
	Week 8	Peg	21	17 (81.0)	12.75 (19.12)	0.0	0.00	50.0		
		SoC	8	6 (75.0)	13.89 (16.39)	0.0	8.33	33.3		
	Week 12	Peg	21	18 (85.7)	13.89 (23.04)	0.0	0.00	66.7		
		SoC	8	6 (75.0)	25.00 (27.39)	0.0	16.67	66.7		
	Week 16	Peg	21	17 (81.0)	12.75 (17.21)	0.0	16.67	66.7		
		SoC	8	7 (87.5)	19.05 (31.07)	0.0	0.00	83.3		
	Week 20	Peg	21	18 (85.7)	13.89 (22.32)	0.0	0.00	66.7		
		SoC	8	7 (87.5)	16.67 (25.46)	0.0	0.00	66.7		
	Week 26	Peg	21	15 (71.4)	13.33 (19.11)	0.0	0.00	50.0		
		SoC	8	4 (50.0)	8.33 (9.62)	0.0	8.33	16.7		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SIHA: Change from baseline in QLQ-C30 Pain (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Pain (censored)	>= 4	Baseline	Peg	14	14 (100.0)	8.33 (18.20)	0.0	0.00	66.7	
			SoC	10	10 (100.0)	18.33 (27.72)	0.0	8.33	83.3	
		Week 4	Peg	14	13 (92.9)	19.23 (24.39)	0.0	16.67	83.3	
			SoC	10	10 (100.0)	21.67 (26.12)	0.0	16.67	83.3	
		Week 8	Peg	14	12 (85.7)	13.89 (19.89)	0.0	8.33	66.7	
			SoC	10	10 (100.0)	21.67 (19.33)	0.0	16.67	50.0	
		Week 12	Peg	14	13 (92.9)	11.54 (14.25)	0.0	0.00	33.3	
			SoC	10	10 (100.0)	20.00 (18.92)	0.0	25.00	50.0	
		Week 16	Peg	14	13 (92.9)	19.23 (24.39)	0.0	16.67	83.3	
			SoC	10	10 (100.0)	20.00 (18.92)	0.0	25.00	50.0	
		Week 20	Peg	14	14 (100.0)	14.29 (25.20)	0.0	0.00	66.7	
			SoC	10	10 (100.0)	23.33 (17.92)	0.0	33.33	50.0	
		Week 26	Peg	14	12 (85.7)	19.44 (22.29)	0.0	16.67	66.7	
			SoC	10	9 (90.0)	16.67 (16.67)	0.0	16.67	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SIHA: Change from baseline in QLQ-C30 Pain (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in QLQ-C30 Pain (censored)	Week 4	Peg	21	18 (85.7)	-3.70 (22.55)	-33.3	0.00	50.0	0.60 (0.54) [-0.41, 1.73]
		SoC	8	5 (62.5)	-16.67 (11.79)	-33.3	-16.67	0.0	
	Week 8	Peg	21	17 (81.0)	-1.96 (19.44)	-33.3	0.00	50.0	0.75 (0.55) [-0.26, 1.93]
		SoC	8	5 (62.5)	-16.67 (16.67)	-33.3	-16.67	0.0	
	Week 12	Peg	21	18 (85.7)	-1.85 (26.75)	-50.0	0.00	66.7	0.06 (0.53) [-0.99, 1.11]
		SoC	8	5 (62.5)	-3.33 (21.73)	-16.7	-16.67	33.3	
	Week 16	Peg	21	17 (81.0)	-2.94 (27.79)	-50.0	0.00	66.7	0.27 (0.50) [-0.69, 1.29]
		SoC	8	6 (75.0)	-11.11 (32.77)	-33.3	-25.00	50.0	
	Week 20	Peg	21	18 (85.7)	-3.70 (28.90)	-50.0	0.00	66.7	0.21 (0.53) [-0.82, 1.29]
		SoC	8	5 (62.5)	-10.00 (27.89)	-33.3	-16.67	33.3	
	Week 26	Peg	21	15 (71.4)	-3.33 (20.12)	-33.3	0.00	50.0	1.45 (0.74) [0.21, 3.11]
		SoC	8	3 (37.5)	-33.33 (16.67)	-50.0	-33.33	-16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWC_SIHA: Change from baseline in QLQ-C30 Pain (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in QLQ-C30 Pain (censored)	Week 4	Peg	14	13 (92.9)	10.26 (28.50)	-33.3	0.00	83.3	0.27 (0.44) [-0.58, 1.18]
		SoC	10	10 (100.0)	3.33 (18.92)	-33.3	0.00	33.3	
	Week 8	Peg	14	12 (85.7)	4.17 (27.64)	-50.0	0.00	66.7	0.03 (0.45) [-0.86, 0.93]
		SoC	10	10 (100.0)	3.33 (23.31)	-33.3	0.00	33.3	
	Week 12	Peg	14	13 (92.9)	2.56 (27.93)	-66.7	0.00	33.3	0.03 (0.44) [-0.84, 0.91]
		SoC	10	10 (100.0)	1.67 (25.40)	-50.0	8.33	33.3	
	Week 16	Peg	14	13 (92.9)	10.26 (35.71)	-66.7	0.00	83.3	0.26 (0.44) [-0.59, 1.17]
		SoC	10	10 (100.0)	1.67 (25.40)	-50.0	8.33	33.3	
	Week 20	Peg	14	14 (100.0)	5.95 (23.21)	-16.7	0.00	66.7	0.04 (0.43) [-0.81, 0.91]
		SoC	10	10 (100.0)	5.00 (19.33)	-33.3	8.33	33.3	
	Week 26	Peg	14	12 (85.7)	9.72 (27.94)	-33.3	0.00	66.7	0.15 (0.47) [-0.75, 1.10]
		SoC	10	9 (90.0)	5.56 (23.57)	-50.0	16.67	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SICC: Change from baseline in QLQ-C30 Fatigue (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.6761

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SICC: Change from baseline in QLQ-C30 Fatigue (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 60	Peg	Week 4	29	26 (89.7)	-15.57 (2.78)	(-21.22, -9.92)	-14.81 (5.16)	(-25.30, -4.32)	0.0071	*
		Week 8	29	24 (82.8)	-19.06 (3.43)	(-26.06, -12.06)	-21.31 (6.21)	(-33.97, -8.66)	0.0017	*
		Week 12	29	26 (89.7)	-17.91 (3.07)	(-24.14, -11.67)	-19.63 (5.69)	(-31.20, -8.06)	0.0015	*
		Week 16	29	25 (86.2)	-21.36 (3.77)	(-29.04, -13.68)	-17.82 (6.65)	(-31.35, -4.28)	0.0115	*
		Week 20	29	27 (93.1)	-20.80 (3.58)	(-28.07, -13.52)	-20.33 (6.73)	(-34.00, -6.65)	0.0048	*
	Week 26	29	23 (79.3)	-20.09 (3.46)	(-27.18, -13.01)	-17.37 (6.45)	(-30.56, -4.18)	0.0116	*	
	SoC	Week 4	13	11 (84.6)	-0.76 (4.29)	(-9.48, 7.96)				
		Week 8	13	11 (84.6)	2.25 (5.09)	(-8.13, 12.63)				
		Week 12	13	11 (84.6)	1.72 (4.73)	(-7.90, 11.34)				
		Week 16	13	12 (92.3)	-3.54 (5.45)	(-14.62, 7.54)				
		Week 20	13	11 (84.6)	-0.47 (5.65)	(-11.94, 11.00)				
Week 26	13	10 (76.9)	-2.72 (5.35)	(-13.67, 8.23)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SICC: Change from baseline in QLQ-C30 Fatigue (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	-15.57 (12.12)	(-46.72, 15.58)	-27.50 (19.36)	(-77.26, 22.26)	0.2147	
		Week 8	6	5 (83.3)	-17.98 (12.99)	(-51.37, 15.40)	-28.00 (20.75)	(-81.34, 25.33)	0.2350	
		Week 12	6	5 (83.3)	-7.45 (13.14)	(-41.23, 26.34)	-22.25 (21.00)	(-76.22, 31.73)	0.3378	
		Week 16	6	5 (83.3)	-12.15 (13.77)	(-47.55, 23.25)	-24.10 (21.89)	(-80.36, 32.17)	0.3212	
		Week 20	6	5 (83.3)	-14.58 (13.25)	(-48.63, 19.47)	-27.19 (21.06)	(-81.32, 26.94)	0.2531	
		Week 26	6	4 (66.7)	-20.31 (5.52)	(-44.05, 3.44)	-0.00 (11.02)	(-47.40, 47.40)	1.0000	
	SoC	Week 4	5	4 (80.0)	11.93 (14.21)	(-24.60, 48.46)				
		Week 8	5	4 (80.0)	10.02 (15.23)	(-29.13, 49.17)				
		Week 12	5	4 (80.0)	14.80 (15.41)	(-24.82, 54.42)				
		Week 16	5	4 (80.0)	11.94 (16.18)	(-29.64, 53.52)				
		Week 20	5	4 (80.0)	12.61 (15.56)	(-27.39, 52.61)				
		Week 26	5	2 (40.0)	-20.31 (9.55)	(-61.38, 20.77)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SIHC: Change from baseline in QLQ-C30 Fatigue (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Fatigue (censored)	< 60	Baseline	Peg	29	29 (100.0)	39.46 (21.12)	0.0	33.33	100.0	
			SoC	13	12 (92.3)	37.04 (20.83)	0.0	38.89	66.7	
		Week 4	Peg	29	26 (89.7)	23.08 (14.03)	0.0	22.22	44.4	
			SoC	13	12 (92.3)	35.19 (15.59)	11.1	33.33	66.7	
		Week 8	Peg	29	24 (82.8)	19.91 (17.33)	0.0	16.67	66.7	
			SoC	13	11 (84.6)	40.40 (16.68)	11.1	33.33	66.7	
		Week 12	Peg	29	26 (89.7)	20.09 (16.64)	0.0	16.67	66.7	
			SoC	13	11 (84.6)	38.38 (14.37)	11.1	33.33	66.7	
		Week 16	Peg	29	25 (86.2)	16.44 (20.57)	0.0	11.11	88.9	
			SoC	13	12 (92.3)	34.26 (16.04)	11.1	33.33	66.7	
		Week 20	Peg	29	27 (93.1)	17.28 (19.33)	0.0	11.11	77.8	
			SoC	13	12 (92.3)	35.19 (16.30)	11.1	33.33	66.7	
		Week 26	Peg	29	23 (79.3)	18.36 (17.28)	0.0	22.22	66.7	
			SoC	13	10 (76.9)	35.56 (13.66)	11.1	33.33	66.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SIHC: Change from baseline in QLQ-C30 Fatigue (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Fatigue (censored)	>= 60	Baseline	Peg	6	6 (100.0)	31.48 (29.33)	0.0	27.78	77.8	
			SoC	5	4 (80.0)	41.67 (24.64)	22.2	33.33	77.8	
	Week 4		Peg	6	5 (83.3)	15.56 (16.85)	0.0	11.11	33.3	
			SoC	5	5 (100.0)	42.22 (38.01)	0.0	33.33	100.0	
	Week 8		Peg	6	5 (83.3)	13.33 (14.49)	0.0	11.11	33.3	
			SoC	5	5 (100.0)	40.00 (39.75)	0.0	33.33	100.0	
	Week 12		Peg	6	5 (83.3)	24.44 (14.49)	0.0	33.33	33.3	
			SoC	5	5 (100.0)	42.22 (41.13)	0.0	33.33	100.0	
	Week 16		Peg	6	5 (83.3)	24.44 (18.26)	0.0	33.33	44.4	
			SoC	5	5 (100.0)	42.22 (41.13)	0.0	33.33	100.0	
	Week 20		Peg	6	5 (83.3)	22.22 (17.57)	0.0	22.22	44.4	
			SoC	5	5 (100.0)	42.22 (41.13)	0.0	33.33	100.0	
	Week 26		Peg	6	4 (66.7)	11.11 (12.83)	0.0	11.11	22.2	
			SoC	5	3 (60.0)	14.81 (16.97)	0.0	11.11	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SIHC: Change from baseline in QLQ-C30 Fatigue (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in QLQ-C30 Fatigue (censored)	Week 4	Peg	29	26 (89.7)	-15.81 (18.64)	-66.7	-22.22	22.2	-0.79 (0.38) [-1.60, -0.09]
		SoC	13	11 (84.6)	0.00 (21.66)	-33.3	0.00	33.3	
	Week 8	Peg	29	24 (82.8)	-19.91 (21.23)	-66.7	-22.22	22.2	-1.13 (0.40) [-2.00, -0.41]
		SoC	13	11 (84.6)	4.04 (19.42)	-33.3	0.00	33.3	
	Week 12	Peg	29	26 (89.7)	-17.95 (20.39)	-77.8	-22.22	11.1	-0.94 (0.39) [-1.76, -0.23]
		SoC	13	11 (84.6)	2.02 (22.12)	-33.3	0.00	33.3	
	Week 16	Peg	29	25 (86.2)	-21.78 (23.67)	-88.9	-22.22	44.4	-0.82 (0.38) [-1.62, -0.13]
		SoC	13	12 (92.3)	-2.78 (20.17)	-33.3	-11.11	33.3	
	Week 20	Peg	29	27 (93.1)	-20.99 (24.13)	-88.9	-22.22	33.3	-0.88 (0.38) [-1.69, -0.18]
		SoC	13	11 (84.6)	0.00 (21.08)	-33.3	0.00	33.3	
Week 26	Peg	29	23 (79.3)	-20.77 (23.04)	-88.9	-22.22	22.2	-0.83 (0.41) [-1.70, -0.09]	
	SoC	13	10 (76.9)	-1.11 (23.10)	-33.3	-5.56	33.3		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SIHC: Change from baseline in QLQ-C30 Fatigue (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in QLQ-C30 Fatigue (censored)	Week 4	Peg	6	5 (83.3)	-13.33 (38.01)	-77.8	0.00	22.2	-0.71 (0.84) [-2.64, 0.67]
		SoC	5	4 (80.0)	11.11 (15.71)	-11.1	16.67	22.2	
	Week 8	Peg	6	5 (83.3)	-15.56 (35.66)	-77.8	0.00	11.1	-0.70 (0.83) [-2.63, 0.68]
		SoC	5	4 (80.0)	8.33 (21.03)	-22.2	16.67	22.2	
	Week 12	Peg	6	5 (83.3)	-4.44 (32.01)	-55.6	0.00	22.2	-0.48 (0.81) [-2.28, 0.95]
		SoC	5	4 (80.0)	11.11 (24.00)	-22.2	16.67	33.3	
	Week 16	Peg	6	5 (83.3)	-11.11 (33.33)	-66.7	0.00	22.2	-0.67 (0.83) [-2.57, 0.72]
		SoC	5	4 (80.0)	11.11 (24.00)	-22.2	16.67	33.3	
	Week 20	Peg	6	5 (83.3)	-13.33 (30.83)	-66.7	0.00	11.1	-0.77 (0.84) [-2.74, 0.60]
		SoC	5	4 (80.0)	11.11 (24.00)	-22.2	16.67	33.3	
	Week 26	Peg	6	4 (66.7)	-22.22 (39.54)	-77.8	-11.11	11.1	-0.37 (1.25) [-3.11, 1.77]
		SoC	5	2 (40.0)	-5.56 (23.57)	-22.2	-5.56	11.1	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SICD: Change from baseline in QLQ-C30 Fatigue (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA				
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA	
Sex	Peg	Overall									0.9235

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SICD: Change from baseline in QLQ-C30 Fatigue (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Male	Peg	Week 4	19	16 (84.2)	-9.28 (3.27)	(-16.11, -2.46)	-15.50 (5.65)	(-27.28, -3.73)	0.0125	*
		Week 8	19	15 (78.9)	-14.11 (4.21)	(-22.93, -5.29)	-23.61 (7.16)	(-38.60, -8.62)	0.0038	*
		Week 12	19	17 (89.5)	-8.03 (3.68)	(-15.69, -0.37)	-17.15 (6.54)	(-30.74, -3.55)	0.0159	*
		Week 16	19	16 (84.2)	-13.66 (5.16)	(-24.40, -2.92)	-19.13 (8.72)	(-37.26, -1.00)	0.0396	*
		Week 20	19	17 (89.5)	-11.24 (4.74)	(-21.11, -1.38)	-16.32 (8.41)	(-33.81, 1.17)	0.0659	
		Week 26	19	14 (73.7)	-14.49 (4.41)	(-23.84, -5.15)	-14.97 (8.05)	(-32.04, 2.09)	0.0814	
	SoC	Week 4	10	8 (80.0)	6.22 (4.65)	(-3.48, 15.92)				
		Week 8	10	8 (80.0)	9.50 (5.82)	(-2.69, 21.69)				
		Week 12	10	8 (80.0)	9.11 (5.42)	(-2.15, 20.38)				
		Week 16	10	9 (90.0)	5.47 (7.02)	(-9.13, 20.08)				
		Week 20	10	8 (80.0)	5.07 (6.98)	(-9.44, 19.59)				
		Week 26	10	6 (60.0)	0.48 (6.73)	(-13.79, 14.76)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SICD: Change from baseline in QLQ-C30 Fatigue (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Female	Peg	Week 4	16	15 (93.8)	-22.26 (5.40)	(-33.60, -10.92)	-22.59 (11.40)	(-46.54, 1.36)	0.0630	
		Week 8	16	14 (87.5)	-23.90 (5.80)	(-36.14, -11.66)	-23.63 (12.19)	(-49.36, 2.09)	0.0694	
		Week 12	16	14 (87.5)	-25.74 (5.66)	(-37.68, -13.81)	-24.68 (11.89)	(-49.77, 0.41)	0.0535	
		Week 16	16	14 (87.5)	-26.97 (5.97)	(-39.56, -14.38)	-21.46 (12.54)	(-47.93, 5.00)	0.1053	
		Week 20	16	15 (93.8)	-29.67 (5.27)	(-40.74, -18.61)	-30.04 (11.12)	(-53.40, -6.68)	0.0146	*
	Week 26	16	13 (81.3)	-24.58 (4.45)	(-34.06, -15.10)	-10.74 (9.54)	(-31.08, 9.60)	0.2780		
	SoC	Week 4	8	7 (87.5)	0.33 (9.44)	(-19.50, 20.16)				
		Week 8	8	7 (87.5)	-0.27 (9.78)	(-20.90, 20.36)				
		Week 12	8	7 (87.5)	-1.06 (9.54)	(-21.19, 19.06)				
		Week 16	8	7 (87.5)	-5.51 (10.06)	(-26.73, 15.72)				
		Week 20	8	7 (87.5)	0.36 (9.21)	(-18.98, 19.71)				
	Week 26	8	6 (75.0)	-13.84 (7.81)	(-30.49, 2.82)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SIHD: Change from baseline in QLQ-C30 Fatigue (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Fatigue (censored)	Male	Baseline	Peg	19	19 (100.0)	32.16 (18.85)	0.0	33.33	66.7	
			SoC	10	9 (90.0)	35.80 (19.86)	0.0	33.33	66.7	
		Week 4	Peg	19	16 (84.2)	24.31 (15.30)	0.0	27.78	44.4	
			SoC	10	9 (90.0)	35.80 (17.37)	0.0	33.33	55.6	
		Week 8	Peg	19	15 (78.9)	19.26 (20.77)	0.0	11.11	66.7	
			SoC	10	9 (90.0)	38.27 (19.33)	0.0	33.33	66.7	
		Week 12	Peg	19	17 (89.5)	24.18 (18.10)	0.0	33.33	66.7	
			SoC	10	9 (90.0)	38.27 (18.52)	0.0	33.33	66.7	
		Week 16	Peg	19	16 (84.2)	20.14 (25.08)	0.0	11.11	88.9	
			SoC	10	10 (100.0)	35.56 (17.99)	0.0	33.33	66.7	
		Week 20	Peg	19	17 (89.5)	23.53 (22.87)	0.0	22.22	77.8	
			SoC	10	9 (90.0)	35.80 (20.62)	0.0	33.33	66.7	
		Week 26	Peg	19	14 (73.7)	19.84 (20.06)	0.0	22.22	66.7	
			SoC	10	7 (70.0)	30.16 (13.93)	0.0	33.33	44.4	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SIHD: Change from baseline in QLQ-C30 Fatigue (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Fatigue (censored)	Female	Baseline	Peg	16	16 (100.0)	45.14 (24.83)	11.1	33.33	100.0	
			SoC	8	7 (87.5)	41.27 (23.76)	11.1	33.33	77.8	
		Week 4	Peg	16	15 (93.8)	19.26 (13.59)	0.0	22.22	44.4	
			SoC	8	8 (100.0)	38.89 (29.70)	11.1	27.78	100.0	
		Week 8	Peg	16	14 (87.5)	18.25 (12.02)	0.0	16.67	33.3	
			SoC	8	7 (87.5)	42.86 (31.71)	11.1	33.33	100.0	
		Week 12	Peg	16	14 (87.5)	16.67 (12.89)	0.0	16.67	44.4	
			SoC	8	7 (87.5)	41.27 (31.89)	11.1	33.33	100.0	
		Week 16	Peg	16	14 (87.5)	15.08 (12.79)	0.0	11.11	44.4	
			SoC	8	7 (87.5)	38.10 (33.86)	11.1	33.33	100.0	
		Week 20	Peg	16	15 (93.8)	11.85 (10.68)	0.0	11.11	33.3	
			SoC	8	8 (100.0)	38.89 (30.28)	11.1	33.33	100.0	
		Week 26	Peg	16	13 (81.3)	14.53 (12.33)	0.0	11.11	33.3	
			SoC	8	6 (75.0)	31.48 (20.39)	11.1	33.33	66.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SIHD: Change from baseline in QLQ-C30 Fatigue (censored) by sex
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Male in QLQ-C30 Fatigue (censored)	Week 4	Peg	19	16 (84.2)	-9.03 (16.34)	-33.3	-11.11	22.2	-0.81 (0.47) [-1.82, 0.04]
		SoC	10	8 (80.0)	5.56 (19.70)	-22.2	5.56	33.3	
	Week 8	Peg	19	15 (78.9)	-14.07 (19.91)	-55.6	-11.11	22.2	-1.17 (0.50) [-2.28, -0.31]
		SoC	10	8 (80.0)	8.33 (15.43)	-11.1	11.11	33.3	
	Week 12	Peg	19	17 (89.5)	-7.19 (18.40)	-44.4	0.00	22.2	-0.78 (0.47) [-1.77, 0.06]
		SoC	10	8 (80.0)	8.33 (21.21)	-22.2	11.11	33.3	
	Week 16	Peg	19	16 (84.2)	-13.19 (24.08)	-55.6	-11.11	44.4	-0.72 (0.45) [-1.68, 0.10]
		SoC	10	9 (90.0)	3.70 (20.03)	-22.2	0.00	33.3	
	Week 20	Peg	19	17 (89.5)	-11.11 (21.15)	-55.6	0.00	33.3	-0.77 (0.47) [-1.76, 0.07]
		SoC	10	8 (80.0)	5.56 (20.57)	-22.2	5.56	33.3	
	Week 26	Peg	19	14 (73.7)	-14.29 (20.64)	-55.6	-11.11	22.2	-0.65 (0.53) [-1.78, 0.32]
		SoC	10	6 (60.0)	0.00 (22.22)	-22.2	-0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SIHD: Change from baseline in QLQ-C30 Fatigue (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Fatigue (censored)	Female	Week 4	Peg	16	15 (93.8)	-22.22 (25.55)	-77.8	-22.22	22.2	-0.87 (0.51) [-1.97, 0.03]
			SoC	8	7 (87.5)	0.00 (22.22)	-33.3	0.00	33.3	
		Week 8	Peg	16	14 (87.5)	-24.60 (26.57)	-77.8	-22.22	0.0	-0.98 (0.52) [-2.13, -0.07]
			SoC	8	7 (87.5)	1.59 (23.51)	-33.3	0.00	33.3	
		Week 12	Peg	16	14 (87.5)	-26.19 (23.31)	-77.8	-22.22	11.1	-1.07 (0.53) [-2.24, -0.16]
			SoC	8	7 (87.5)	0.00 (24.00)	-33.3	0.00	33.3	
		Week 16	Peg	16	14 (87.5)	-27.78 (24.94)	-88.9	-22.22	11.1	-0.96 (0.52) [-2.10, -0.05]
			SoC	8	7 (87.5)	-3.17 (23.76)	-33.3	-11.11	33.3	
		Week 20	Peg	16	15 (93.8)	-29.63 (25.77)	-88.9	-22.22	22.2	-1.13 (0.52) [-2.29, -0.23]
			SoC	8	7 (87.5)	0.00 (24.00)	-33.3	0.00	33.3	
		Week 26	Peg	16	13 (81.3)	-28.21 (28.19)	-88.9	-22.22	11.1	-0.87 (0.55) [-2.08, 0.10]
			SoC	8	6 (75.0)	-3.70 (24.00)	-33.3	-5.56	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SICB: Change from baseline in QLQ-C30 Fatigue (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.2207

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SICB: Change from baseline in QLQ-C30 Fatigue (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Latin America	Peg	Week 4	12	11 (91.7)	-24.31 (3.95)	(-33.23, -15.38)	-19.42 (10.41)	(-42.98, 4.13)	0.0950	
		Week 8	12	10 (83.3)	-34.80 (2.17)	(-39.82, -29.79)	-31.04 (5.55)	(-43.83, -18.25)	0.0005	*
		Week 12	12	11 (91.7)	-22.16 (3.32)	(-29.66, -14.66)	-16.56 (8.75)	(-36.35, 3.24)	0.0910	
		Week 16	12	10 (83.3)	-32.49 (3.32)	(-40.15, -24.83)	-28.29 (8.47)	(-47.82, -8.76)	0.0102	*
		Week 20	12	10 (83.3)	-32.71 (3.89)	(-41.69, -23.73)	-24.06 (10.12)	(-47.38, -0.73)	0.0447	*
		Week 26	12	9 (75.0)	-32.17 (5.34)	(-44.79, -19.55)	-22.77 (13.27)	(-54.16, 8.61)	0.1299	
	SoC	Week 4	2	2 (100.0)	-4.88 (9.53)	(-26.44, 16.68)				
		Week 8	2	2 (100.0)	-3.76 (5.02)	(-15.35, 7.82)				
		Week 12	2	2 (100.0)	-5.60 (8.01)	(-23.72, 12.51)				
		Week 16	2	2 (100.0)	-4.20 (7.67)	(-21.90, 13.49)				
		Week 20	2	2 (100.0)	-8.66 (9.13)	(-29.71, 12.40)				
		Week 26	2	2 (100.0)	-9.39 (11.90)	(-37.53, 18.74)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SICB: Change from baseline in QLQ-C30 Fatigue (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Asia Pacific Peg		Week 4	23	20 (87.0)	-10.03 (3.66)	(-17.51, -2.54)	-13.56 (6.04)	(-25.91, -1.21)	0.0326	*
		Week 8	23	19 (82.6)	-9.64 (3.94)	(-17.70, -1.57)	-14.32 (6.39)	(-27.42, -1.23)	0.0332	*
		Week 12	23	20 (87.0)	-12.25 (4.18)	(-20.80, -3.71)	-18.51 (6.91)	(-32.65, -4.37)	0.0121	*
		Week 16	23	20 (87.0)	-12.75 (4.64)	(-22.22, -3.28)	-12.71 (7.41)	(-27.84, 2.41)	0.0964	
		Week 20	23	22 (95.7)	-13.25 (4.26)	(-21.93, -4.56)	-17.02 (7.25)	(-31.81, -2.23)	0.0255	*
		Week 26	23	18 (78.3)	-13.74 (3.64)	(-21.25, -6.24)	-10.51 (6.49)	(-23.90, 2.88)	0.1184	
SoC		Week 4	16	13 (81.3)	3.53 (4.63)	(-5.94, 13.00)				
		Week 8	16	13 (81.3)	4.68 (4.81)	(-5.17, 14.54)				
		Week 12	16	13 (81.3)	6.26 (5.29)	(-4.56, 17.07)				
		Week 16	16	14 (87.5)	-0.04 (5.59)	(-11.44, 11.37)				
		Week 20	16	13 (81.3)	3.77 (5.66)	(-7.77, 15.32)				
		Week 26	16	10 (62.5)	-3.24 (5.22)	(-14.01, 7.54)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SIHb: Change from baseline in QLQ-C30 Fatigue (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Fatigue (censored)	Latin America	Baseline	Peg	12	12 (100.0)	47.22 (30.01)	0.0	38.89	100.0	
			SoC	2	2 (100.0)	22.22 (31.43)	0.0	22.22	44.4	
		Week 4	Peg	12	11 (91.7)	15.15 (12.45)	0.0	11.11	33.3	
			SoC	2	2 (100.0)	33.33 (0.00)	33.3	33.33	33.3	
		Week 8	Peg	12	10 (83.3)	5.56 (7.86)	0.0	0.00	22.2	
			SoC	2	2 (100.0)	33.33 (0.00)	33.3	33.33	33.3	
		Week 12	Peg	12	11 (91.7)	17.17 (10.38)	0.0	11.11	33.3	
			SoC	2	2 (100.0)	33.33 (0.00)	33.3	33.33	33.3	
		Week 16	Peg	12	10 (83.3)	7.78 (11.77)	0.0	0.00	33.3	
			SoC	2	2 (100.0)	33.33 (0.00)	33.3	33.33	33.3	
		Week 20	Peg	12	10 (83.3)	8.89 (11.48)	0.0	5.56	33.3	
			SoC	2	2 (100.0)	33.33 (0.00)	33.3	33.33	33.3	
		Week 26	Peg	12	9 (75.0)	9.88 (15.16)	0.0	0.00	33.3	
			SoC	2	2 (100.0)	33.33 (0.00)	33.3	33.33	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SIHB: Change from baseline in QLQ-C30 Fatigue (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Fatigue (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	33.33 (16.07)	0.0	33.33	66.7	
			SoC	16	14 (87.5)	40.48 (19.78)	11.1	33.33	77.8	
		Week 4	Peg	23	20 (87.0)	25.56 (14.46)	0.0	27.78	44.4	
			SoC	16	15 (93.8)	37.78 (24.77)	0.0	33.33	100.0	
		Week 8	Peg	23	19 (82.6)	25.73 (16.17)	0.0	22.22	66.7	
			SoC	16	14 (87.5)	41.27 (26.30)	0.0	33.33	100.0	
		Week 12	Peg	23	20 (87.0)	22.78 (18.55)	0.0	22.22	66.7	
			SoC	16	14 (87.5)	40.48 (26.01)	0.0	33.33	100.0	
		Week 16	Peg	23	20 (87.0)	22.78 (21.77)	0.0	16.67	88.9	
			SoC	16	15 (93.8)	37.04 (26.45)	0.0	33.33	100.0	
		Week 20	Peg	23	22 (95.7)	22.22 (20.29)	0.0	11.11	77.8	
			SoC	16	15 (93.8)	37.78 (26.49)	0.0	33.33	100.0	
		Week 26	Peg	23	18 (78.3)	20.99 (16.56)	0.0	22.22	66.7	
			SoC	16	11 (68.8)	30.30 (17.98)	0.0	33.33	66.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SIHB: Change from baseline in QLQ-C30 Fatigue (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Fatigue (censored)	Latin America	Week 4	Peg	12	11 (91.7)	-27.27 (27.38)	-77.8	-22.22	11.1	-1.29 (0.91) [-3.38, 0.23]
			SoC	2	2 (100.0)	11.11 (31.43)	-11.1	11.11	33.3	
		Week 8	Peg	12	10 (83.3)	-37.78 (24.12)	-77.8	-33.33	0.0	-1.81 (1.00) [-4.22, -0.29]
			SoC	2	2 (100.0)	11.11 (31.43)	-11.1	11.11	33.3	
		Week 12	Peg	12	11 (91.7)	-25.25 (27.71)	-77.8	-22.22	11.1	-1.21 (0.90) [-3.27, 0.31]
			SoC	2	2 (100.0)	11.11 (31.43)	-11.1	11.11	33.3	
		Week 16	Peg	12	10 (83.3)	-35.56 (27.62)	-88.9	-33.33	0.0	-1.54 (0.96) [-3.82, -0.02]
			SoC	2	2 (100.0)	11.11 (31.43)	-11.1	11.11	33.3	
		Week 20	Peg	12	10 (83.3)	-36.67 (28.23)	-88.9	-33.33	0.0	-1.54 (0.97) [-3.83, -0.03]
			SoC	2	2 (100.0)	11.11 (31.43)	-11.1	11.11	33.3	
		Week 26	Peg	12	9 (75.0)	-37.04 (31.91)	-88.9	-33.33	0.0	-1.38 (0.98) [-3.70, 0.16]
			SoC	2	2 (100.0)	11.11 (31.43)	-11.1	11.11	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SIHB: Change from baseline in QLQ-C30 Fatigue (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Fatigue (censored)	Asia	Week 4	Peg	23	20 (87.0)	-8.89 (15.55)	-33.3	-11.11	22.2	-0.60 (0.38) [-1.39, 0.10]
			SoC	16	13 (81.3)	1.71 (19.69)	-33.3	0.00	33.3	
	Pacific	Week 8	Peg	23	19 (82.6)	-9.36 (16.67)	-44.4	0.00	22.2	-0.76 (0.39) [-1.58, -0.06]
			SoC	16	13 (81.3)	4.27 (18.45)	-33.3	11.11	33.3	
		Week 12	Peg	23	20 (87.0)	-10.56 (17.83)	-44.4	-11.11	22.2	-0.70 (0.38) [-1.50, -0.00]
			SoC	16	13 (81.3)	3.42 (21.94)	-33.3	11.11	33.3	
		Week 16	Peg	23	20 (87.0)	-12.22 (20.36)	-44.4	-16.67	44.4	-0.54 (0.37) [-1.31, 0.14]
			SoC	16	14 (87.5)	-0.79 (20.66)	-33.3	-5.56	33.3	
		Week 20	Peg	23	22 (95.7)	-12.12 (19.37)	-44.4	-22.22	33.3	-0.67 (0.37) [-1.45, 0.01]
			SoC	16	13 (81.3)	1.71 (21.20)	-33.3	0.00	33.3	
		Week 26	Peg	23	18 (78.3)	-12.96 (16.72)	-33.3	-11.11	22.2	-0.45 (0.42) [-1.31, 0.33]
			SoC	16	10 (62.5)	-4.44 (21.08)	-33.3	-5.56	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SICA: Change from baseline in QLQ-C30 Fatigue (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.5425

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SICA: Change from baseline in QLQ-C30 Fatigue (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 4	21	18 (85.7)	-15.37 (3.49)	(-22.65, -8.09)	-17.37 (7.50)	(-33.01, -1.73)	0.0312	*
		Week 8	21	17 (81.0)	-20.76 (3.56)	(-28.22, -13.30)	-24.66 (7.48)	(-40.33, -8.99)	0.0038	*
		Week 12	21	18 (85.7)	-16.55 (4.07)	(-25.04, -8.05)	-27.22 (8.74)	(-45.45, -8.99)	0.0055	*
		Week 16	21	17 (81.0)	-24.49 (4.03)	(-32.89, -16.09)	-27.22 (7.89)	(-43.67, -10.77)	0.0025	*
		Week 20	21	18 (85.7)	-20.35 (4.46)	(-29.65, -11.05)	-24.71 (9.56)	(-44.65, -4.76)	0.0177	*
	Week 26	21	15 (71.4)	-25.18 (3.80)	(-33.28, -17.09)	-21.46 (9.32)	(-41.33, -1.59)	0.0361	*	
	SoC	Week 4	8	5 (62.5)	2.00 (6.63)	(-11.83, 15.83)				
		Week 8	8	5 (62.5)	3.90 (6.58)	(-9.86, 17.67)				
		Week 12	8	5 (62.5)	10.68 (7.73)	(-5.45, 26.80)				
		Week 16	8	6 (75.0)	2.73 (6.78)	(-11.41, 16.87)				
		Week 20	8	5 (62.5)	4.36 (8.46)	(-13.29, 22.00)				
Week 26	8	3 (37.5)	-3.72 (8.51)	(-21.86, 14.41)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SICA: Change from baseline in QLQ-C30 Fatigue (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	-16.06 (4.89)	(-26.26, -5.86)	-20.27 (7.41)	(-35.74, -4.81)	0.0128	*
		Week 8	14	12 (85.7)	-17.16 (6.03)	(-29.78, -4.55)	-23.31 (8.95)	(-42.04, -4.58)	0.0174	*
		Week 12	14	13 (92.9)	-15.58 (5.06)	(-26.13, -5.02)	-18.05 (7.68)	(-34.06, -2.04)	0.0291	*
		Week 16	14	13 (92.9)	-14.68 (6.52)	(-28.28, -1.07)	-14.87 (9.89)	(-35.50, 5.76)	0.1484	
		Week 20	14	14 (100.0)	-19.39 (5.60)	(-31.05, -7.74)	-22.10 (8.69)	(-40.16, -4.04)	0.0189	*
	Week 26	14	12 (85.7)	-15.45 (5.06)	(-26.08, -4.82)	-13.84 (7.76)	(-30.13, 2.46)	0.0913		
	SoC	Week 4	10	10 (100.0)	4.21 (5.57)	(-7.41, 15.84)				
		Week 8	10	10 (100.0)	6.15 (6.60)	(-7.67, 19.97)				
		Week 12	10	10 (100.0)	2.47 (5.77)	(-9.56, 14.51)				
		Week 16	10	10 (100.0)	0.19 (7.44)	(-15.32, 15.70)				
		Week 20	10	10 (100.0)	2.71 (6.63)	(-11.08, 16.50)				
Week 26		10	9 (90.0)	-1.62 (5.85)	(-13.90, 10.67)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SIHA: Change from baseline in QLQ-C30 Fatigue (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Fatigue (censored)	< 4	Baseline	Peg	21	21 (100.0)	40.21 (26.17)	0.0	33.33	100.0	
			SoC	8	6 (75.0)	40.74 (15.18)	22.2	38.89	66.7	
		Week 4	Peg	21	18 (85.7)	22.22 (15.24)	0.0	22.22	44.4	
			SoC	8	7 (87.5)	31.75 (18.62)	0.0	33.33	55.6	
		Week 8	Peg	21	17 (81.0)	16.99 (15.77)	0.0	11.11	55.6	
			SoC	8	6 (75.0)	35.19 (20.39)	0.0	33.33	55.6	
		Week 12	Peg	21	18 (85.7)	20.99 (18.63)	0.0	22.22	66.7	
			SoC	8	6 (75.0)	40.74 (22.95)	0.0	44.44	66.7	
		Week 16	Peg	21	17 (81.0)	15.03 (17.10)	0.0	11.11	44.4	
			SoC	8	7 (87.5)	36.51 (21.96)	0.0	33.33	66.7	
		Week 20	Peg	21	18 (85.7)	19.75 (18.10)	0.0	11.11	55.6	
			SoC	8	7 (87.5)	34.92 (24.37)	0.0	33.33	66.7	
		Week 26	Peg	21	15 (71.4)	15.56 (15.02)	0.0	11.11	44.4	
			SoC	8	4 (50.0)	27.78 (19.25)	0.0	33.33	44.4	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SIHA: Change from baseline in QLQ-C30 Fatigue (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Fatigue (censored)	>= 4	Baseline	Peg	14	14 (100.0)	34.92 (15.63)	0.0	33.33	55.6	
			SoC	10	10 (100.0)	36.67 (24.60)	0.0	33.33	77.8	
	Week 4	Peg	14	13 (92.9)	21.37 (13.95)	0.0	22.22	44.4		
		SoC	10	10 (100.0)	41.11 (26.22)	11.1	33.33	100.0		
	Week 8	Peg	14	12 (85.7)	21.30 (18.63)	0.0	16.67	66.7		
		SoC	10	10 (100.0)	43.33 (27.44)	11.1	33.33	100.0		
	Week 12	Peg	14	13 (92.9)	20.51 (12.71)	0.0	22.22	44.4		
		SoC	10	10 (100.0)	38.89 (26.32)	11.1	33.33	100.0		
	Week 16	Peg	14	13 (92.9)	21.37 (23.77)	0.0	11.11	88.9		
		SoC	10	10 (100.0)	36.67 (27.74)	11.1	33.33	100.0		
	Week 20	Peg	14	14 (100.0)	15.87 (20.31)	0.0	11.11	77.8		
		SoC	10	10 (100.0)	38.89 (26.32)	11.1	33.33	100.0		
	Week 26	Peg	14	12 (85.7)	19.44 (19.03)	0.0	22.22	66.7		
		SoC	10	9 (90.0)	32.10 (16.14)	11.1	33.33	66.7		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SIHA: Change from baseline in QLQ-C30 Fatigue (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in QLQ-C30 Fatigue (censored)	Week 4	Peg	21	18 (85.7)	-14.81 (25.28)	-77.8	-11.11	22.2	-0.58 (0.54) [-1.72, 0.42]
		SoC	8	5 (62.5)	0.00 (20.79)	-22.2	-11.11	22.2	
	Week 8	Peg	21	17 (81.0)	-20.26 (25.23)	-77.8	-11.11	0.0	-0.92 (0.56) [-2.13, 0.08]
		SoC	8	5 (62.5)	2.22 (14.49)	-11.1	0.00	22.2	
	Week 12	Peg	21	18 (85.7)	-16.05 (25.34)	-77.8	-16.67	22.2	-0.99 (0.56) [-2.19, 0.01]
		SoC	8	5 (62.5)	8.89 (19.88)	-11.1	11.11	33.3	
	Week 16	Peg	21	17 (81.0)	-24.18 (25.23)	-88.9	-22.22	11.1	-1.06 (0.53) [-2.22, -0.13]
		SoC	8	6 (75.0)	1.85 (17.80)	-11.1	-5.56	33.3	
	Week 20	Peg	21	18 (85.7)	-20.37 (27.81)	-88.9	-22.22	22.2	-0.91 (0.55) [-2.10, 0.08]
		SoC	8	5 (62.5)	4.44 (18.59)	-11.1	0.00	33.3	
	Week 26	Peg	21	15 (71.4)	-24.44 (29.16)	-88.9	-11.11	11.1	-0.58 (0.69) [-2.03, 0.69]
		SoC	8	3 (37.5)	-7.41 (16.97)	-22.2	-11.11	11.1	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QFWC_SIHA: Change from baseline in QLQ-C30 Fatigue (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in QLQ-C30 Fatigue (censored)	Week 4	Peg	14	13 (92.9)	-16.24 (17.34)	-44.4	-22.22	22.2	-1.05 (0.48) [-2.10, -0.22]
		SoC	10	10 (100.0)	4.44 (21.08)	-33.3	0.00	33.3	
	Week 8	Peg	14	12 (85.7)	-17.59 (21.95)	-44.4	-22.22	22.2	-1.07 (0.49) [-2.15, -0.23]
		SoC	10	10 (100.0)	6.67 (21.72)	-33.3	11.11	33.3	
	Week 12	Peg	14	13 (92.9)	-15.38 (19.00)	-44.4	-11.11	22.2	-0.80 (0.46) [-1.80, 0.03]
		SoC	10	10 (100.0)	2.22 (23.89)	-33.3	5.56	33.3	
	Week 16	Peg	14	13 (92.9)	-14.53 (25.00)	-44.4	-22.22	44.4	-0.57 (0.45) [-1.53, 0.26]
		SoC	10	10 (100.0)	0.00 (24.00)	-33.3	-5.56	33.3	
	Week 20	Peg	14	14 (100.0)	-19.05 (21.54)	-44.4	-22.22	33.3	-0.91 (0.46) [-1.91, -0.10]
		SoC	10	10 (100.0)	2.22 (23.89)	-33.3	5.56	33.3	
	Week 26	Peg	14	12 (85.7)	-16.67 (19.25)	-33.3	-22.22	22.2	-0.74 (0.49) [-1.79, 0.12]
		SoC	10	9 (90.0)	0.00 (24.22)	-33.3	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SICC: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.8545

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SICC: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 60	Peg	Week 4	29	26 (89.7)	-4.77 (1.09)	(-6.98, -2.57)	-5.47 (2.02)	(-9.58, -1.36)	0.0107	*
		Week 8	29	24 (82.8)	-4.94 (1.38)	(-7.75, -2.13)	-5.23 (2.50)	(-10.32, -0.14)	0.0445	*
		Week 12	29	26 (89.7)	-2.02 (2.17)	(-6.43, 2.40)	-3.76 (4.04)	(-11.98, 4.45)	0.3582	
		Week 16	29	25 (86.2)	-2.10 (2.06)	(-6.29, 2.10)	-3.88 (3.66)	(-11.33, 3.56)	0.2961	
		Week 20	29	27 (93.1)	-0.29 (2.58)	(-5.53, 4.95)	0.52 (4.86)	(-9.35, 10.39)	0.9159	
		Week 26	29	23 (79.3)	-0.65 (2.42)	(-5.59, 4.29)	1.17 (4.50)	(-8.03, 10.38)	0.7960	
	SoC	Week 4	13	11 (84.6)	0.69 (1.68)	(-2.72, 4.11)				
		Week 8	13	11 (84.6)	0.29 (2.04)	(-3.88, 4.46)				
		Week 12	13	11 (84.6)	1.75 (3.35)	(-5.08, 8.57)				
		Week 16	13	12 (92.3)	1.79 (2.99)	(-4.29, 7.86)				
		Week 20	13	11 (84.6)	-0.81 (4.07)	(-9.08, 7.47)				
		Week 26	13	10 (76.9)	-1.83 (3.74)	(-9.47, 5.81)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SICC: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	2.51 (8.81)	(-20.14, 25.17)	-5.36 (14.02)	(-41.40, 30.69)	0.7181	
		Week 8	6	5 (83.3)	1.85 (4.54)	(-9.81, 13.51)	-4.17 (7.22)	(-22.72, 14.38)	0.5887	
		Week 12	6	5 (83.3)	-1.72 (2.90)	(-9.17, 5.73)	-3.57 (4.61)	(-15.42, 8.28)	0.4736	
		Week 16	6	5 (83.3)	-1.72 (2.90)	(-9.17, 5.73)	-3.57 (4.61)	(-15.42, 8.28)	0.4736	
		Week 20	6	5 (83.3)	1.72 (4.15)	(-8.95, 12.39)	-0.60 (6.60)	(-17.56, 16.37)	0.9317	
		Week 26	6	4 (66.7)	-2.78 (NE)	(NE, NE)	NE (NE)	(NE, NE)	NE	
	SoC	Week 4	5	4 (80.0)	7.87 (10.26)	(-18.52, 34.26)				
		Week 8	5	4 (80.0)	6.02 (5.28)	(-7.56, 19.60)				
		Week 12	5	4 (80.0)	1.85 (3.37)	(-6.82, 10.53)				
		Week 16	5	4 (80.0)	1.85 (3.37)	(-6.82, 10.53)				
		Week 20	5	4 (80.0)	2.31 (4.83)	(-10.11, 14.74)				
		Week 26	5	2 (40.0)	-2.78 (NE)	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SIHC: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Nausea/Vomiting (censored)	< 60	Baseline	Peg	29	29 (100.0)	6.90 (14.45)	0.0	0.00	50.0	
			SoC	13	12 (92.3)	2.78 (9.62)	0.0	0.00	33.3	
		Week 4	Peg	29	26 (89.7)	1.28 (4.53)	0.0	0.00	16.7	
			SoC	13	12 (92.3)	5.56 (8.21)	0.0	0.00	16.7	
		Week 8	Peg	29	24 (82.8)	1.39 (4.71)	0.0	0.00	16.7	
			SoC	13	11 (84.6)	6.06 (11.24)	0.0	0.00	33.3	
		Week 12	Peg	29	26 (89.7)	4.49 (13.79)	0.0	0.00	66.7	
			SoC	13	11 (84.6)	6.06 (11.24)	0.0	0.00	33.3	
		Week 16	Peg	29	25 (86.2)	4.00 (11.06)	0.0	0.00	50.0	
			SoC	13	12 (92.3)	6.94 (11.14)	0.0	0.00	33.3	
		Week 20	Peg	29	27 (93.1)	4.94 (14.48)	0.0	0.00	66.7	
			SoC	13	12 (92.3)	4.17 (7.54)	0.0	0.00	16.7	
		Week 26	Peg	29	23 (79.3)	5.07 (12.75)	0.0	0.00	50.0	
			SoC	13	10 (76.9)	3.33 (7.03)	0.0	0.00	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SIHC: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Nausea/Vomiting (censored)	>= 60	Baseline	Peg	6	6 (100.0)	2.78 (6.80)	0.0	0.00	16.7	
			SoC	5	4 (80.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 4	Peg	6	5 (83.3)	3.33 (7.45)	0.0	0.00	16.7		
		SoC	5	5 (100.0)	10.00 (22.36)	0.0	0.00	50.0		
	Week 8	Peg	6	5 (83.3)	3.33 (7.45)	0.0	0.00	16.7		
		SoC	5	5 (100.0)	6.67 (9.13)	0.0	0.00	16.7		
	Week 12	Peg	6	5 (83.3)	0.00 (0.00)	0.0	0.00	0.0		
		SoC	5	5 (100.0)	3.33 (7.45)	0.0	0.00	16.7		
	Week 16	Peg	6	5 (83.3)	0.00 (0.00)	0.0	0.00	0.0		
		SoC	5	5 (100.0)	3.33 (7.45)	0.0	0.00	16.7		
	Week 20	Peg	6	5 (83.3)	3.33 (7.45)	0.0	0.00	16.7		
		SoC	5	5 (100.0)	3.33 (7.45)	0.0	0.00	16.7		
	Week 26	Peg	6	4 (66.7)	0.00 (0.00)	0.0	0.00	0.0		
		SoC	5	3 (60.0)	0.00 (0.00)	0.0	0.00	0.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SIHC: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in QLQ-C30 Nausea/Vomiting (censored)	Week 4	Peg	29	26 (89.7)	-5.77 (13.29)	-50.0	0.00	0.0	-0.69 (0.38) [-1.49, 0.01]
		SoC	13	11 (84.6)	3.03 (10.05)	-16.7	0.00	16.7	
	Week 8	Peg	29	24 (82.8)	-6.25 (14.59)	-50.0	0.00	0.0	-0.71 (0.39) [-1.52, 0.00]
		SoC	13	11 (84.6)	3.03 (6.74)	0.0	0.00	16.7	
	Week 12	Peg	29	26 (89.7)	-2.56 (13.90)	-50.0	0.00	33.3	-0.45 (0.37) [-1.21, 0.26]
		SoC	13	11 (84.6)	3.03 (6.74)	0.0	0.00	16.7	
	Week 16	Peg	29	25 (86.2)	-3.33 (15.96)	-50.0	0.00	33.3	-0.53 (0.37) [-1.29, 0.16]
		SoC	13	12 (92.3)	4.17 (7.54)	0.0	0.00	16.7	
	Week 20	Peg	29	27 (93.1)	-1.23 (20.11)	-50.0	0.00	50.0	-0.14 (0.37) [-0.88, 0.57]
		SoC	13	11 (84.6)	1.52 (13.85)	-33.3	0.00	16.7	
	Week 26	Peg	29	23 (79.3)	-1.45 (17.34)	-50.0	0.00	33.3	-0.09 (0.39) [-0.87, 0.68]
		SoC	13	10 (76.9)	0.00 (13.61)	-33.3	0.00	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SIHC: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in QLQ-C30 Nausea/Vomiting (censored)	Week 4	Peg	6	5 (83.3)	0.00 (11.79)	-16.7	0.00	16.7	-0.60 (0.82) [-2.46, 0.81]
		SoC	5	4 (80.0)	12.50 (25.00)	0.0	0.00	50.0	
	Week 8	Peg	6	5 (83.3)	0.00 (11.79)	-16.7	0.00	16.7	-0.68 (0.83) [-2.59, 0.71]
		SoC	5	4 (80.0)	8.33 (9.62)	0.0	8.33	16.7	
	Week 12	Peg	6	5 (83.3)	-3.33 (7.45)	-16.7	0.00	0.0	-0.85 (0.85) [-2.86, 0.51]
		SoC	5	4 (80.0)	4.17 (8.33)	0.0	0.00	16.7	
	Week 16	Peg	6	5 (83.3)	-3.33 (7.45)	-16.7	0.00	0.0	-0.85 (0.85) [-2.86, 0.51]
		SoC	5	4 (80.0)	4.17 (8.33)	0.0	0.00	16.7	
	Week 20	Peg	6	5 (83.3)	0.00 (11.79)	-16.7	0.00	16.7	-0.35 (0.80) [-2.10, 1.11]
		SoC	5	4 (80.0)	4.17 (8.33)	0.0	0.00	16.7	
	Week 26	Peg	6	4 (66.7)	-4.17 (8.33)	-16.7	0.00	0.0	-0.46 (1.26) [-3.30, 1.62]
		SoC	5	2 (40.0)	0.00 (0.00)	0.0	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SICD: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.2055

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SICD: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Male	Peg	Week 4	19	16 (84.2)	-3.26 (1.68)	(-6.76, 0.24)	-4.08 (2.91)	(-10.16, 1.99)	0.1764	
		Week 8	19	15 (78.9)	-3.45 (2.14)	(-7.92, 1.03)	-6.54 (3.66)	(-14.20, 1.12)	0.0899	
		Week 12	19	17 (89.5)	-0.83 (2.70)	(-6.44, 4.78)	-2.55 (4.80)	(-12.53, 7.44)	0.6014	
		Week 16	19	16 (84.2)	-2.48 (2.72)	(-8.14, 3.17)	-6.09 (4.63)	(-15.72, 3.54)	0.2025	
		Week 20	19	17 (89.5)	2.45 (3.92)	(-5.70, 10.60)	3.73 (6.96)	(-10.74, 18.21)	0.5972	
		Week 26	19	14 (73.7)	1.55 (3.75)	(-6.39, 9.49)	5.18 (6.85)	(-9.33, 19.69)	0.4604	
	SoC	Week 4	10	8 (80.0)	0.82 (2.40)	(-4.18, 5.82)				
		Week 8	10	8 (80.0)	3.09 (2.97)	(-3.12, 9.31)				
		Week 12	10	8 (80.0)	1.72 (3.98)	(-6.56, 9.99)				
		Week 16	10	9 (90.0)	3.61 (3.72)	(-4.13, 11.34)				
		Week 20	10	8 (80.0)	-1.29 (5.77)	(-13.29, 10.71)				
		Week 26	10	6 (60.0)	-3.62 (5.73)	(-15.76, 8.51)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SICD: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Female	Peg	Week 4	16	15 (93.8)	-3.79 (2.88)	(-9.84, 2.27)	-9.52 (6.07)	(-22.28, 3.23)	0.1341	
		Week 8	16	14 (87.5)	-3.97 (1.35)	(-6.81, -1.12)	-4.76 (2.83)	(-10.73, 1.21)	0.1106	
		Week 12	16	14 (87.5)	-3.08 (1.71)	(-6.68, 0.53)	-4.76 (3.58)	(-12.32, 2.80)	0.2016	
		Week 16	16	14 (87.5)	-1.41 (1.99)	(-5.60, 2.78)	-1.43 (4.16)	(-10.21, 7.35)	0.7357	
		Week 20	16	15 (93.8)	-2.40 (1.56)	(-5.68, 0.88)	-1.98 (3.29)	(-8.89, 4.92)	0.5536	
		Week 26	16	13 (81.3)	-3.51 (1.12)	(-5.90, -1.12)	-2.78 (2.38)	(-7.85, 2.29)	0.2611	
	SoC	Week 4	8	7 (87.5)	5.74 (5.03)	(-4.83, 16.31)				
		Week 8	8	7 (87.5)	0.79 (2.27)	(-4.00, 5.58)				
		Week 12	8	7 (87.5)	1.68 (2.88)	(-4.39, 7.75)				
		Week 16	8	7 (87.5)	0.02 (3.34)	(-7.03, 7.07)				
		Week 20	8	7 (87.5)	-0.41 (2.72)	(-6.14, 5.31)				
	Week 26	8	6 (75.0)	-0.73 (1.96)	(-4.90, 3.44)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SIHD: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Nausea/Vomiting (censored)	Male	Baseline	Peg	19	19 (100.0)	6.14 (13.84)	0.0	0.00	50.0	
			SoC	10	9 (90.0)	3.70 (11.11)	0.0	0.00	33.3	
		Week 4	Peg	19	16 (84.2)	3.13 (6.72)	0.0	0.00	16.7	
			SoC	10	9 (90.0)	5.56 (8.33)	0.0	0.00	16.7	
		Week 8	Peg	19	15 (78.9)	3.33 (6.90)	0.0	0.00	16.7	
			SoC	10	9 (90.0)	7.41 (12.11)	0.0	0.00	33.3	
		Week 12	Peg	19	17 (89.5)	5.88 (16.61)	0.0	0.00	66.7	
			SoC	10	9 (90.0)	5.56 (11.79)	0.0	0.00	33.3	
		Week 16	Peg	19	16 (84.2)	4.17 (12.91)	0.0	0.00	50.0	
			SoC	10	10 (100.0)	6.67 (11.65)	0.0	0.00	33.3	
		Week 20	Peg	19	17 (89.5)	7.84 (17.79)	0.0	0.00	66.7	
			SoC	10	9 (90.0)	3.70 (7.35)	0.0	0.00	16.7	
		Week 26	Peg	19	14 (73.7)	8.33 (15.68)	0.0	0.00	50.0	
			SoC	10	7 (70.0)	2.38 (6.30)	0.0	0.00	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SIHD: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Nausea/Vomiting (censored)	Female	Baseline	Peg	16	16 (100.0)	6.25 (13.44)	0.0	0.00	50.0	
			SoC	8	7 (87.5)	0.00 (0.00)	0.0	0.00	0.0	
		Week 4	Peg	16	15 (93.8)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	8	8 (100.0)	8.33 (17.82)	0.0	0.00	50.0	
		Week 8	Peg	16	14 (87.5)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	8	7 (87.5)	4.76 (8.13)	0.0	0.00	16.7	
		Week 12	Peg	16	14 (87.5)	1.19 (4.45)	0.0	0.00	16.7	
			SoC	8	7 (87.5)	4.76 (8.13)	0.0	0.00	16.7	
		Week 16	Peg	16	14 (87.5)	2.38 (6.05)	0.0	0.00	16.7	
			SoC	8	7 (87.5)	4.76 (8.13)	0.0	0.00	16.7	
		Week 20	Peg	16	15 (93.8)	1.11 (4.30)	0.0	0.00	16.7	
			SoC	8	8 (100.0)	4.17 (7.72)	0.0	0.00	16.7	
		Week 26	Peg	16	13 (81.3)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	8	6 (75.0)	2.78 (6.80)	0.0	0.00	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SIHD: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Nausea/Vomiting (censored)	Male	Week 4	Peg	19	16 (84.2)	-4.17 (12.91)	-33.3	0.00	16.7	-0.49 (0.46) [-1.46, 0.37]
			SoC	10	8 (80.0)	2.08 (10.68)	-16.7	0.00	16.7	
		Week 8	Peg	19	15 (78.9)	-4.44 (14.73)	-50.0	0.00	16.7	-0.65 (0.47) [-1.65, 0.22]
			SoC	10	8 (80.0)	4.17 (7.72)	0.0	0.00	16.7	
		Week 12	Peg	19	17 (89.5)	-0.98 (12.46)	-33.3	0.00	33.3	-0.27 (0.45) [-1.19, 0.59]
			SoC	10	8 (80.0)	2.08 (5.89)	0.0	0.00	16.7	
		Week 16	Peg	19	16 (84.2)	-3.13 (15.18)	-33.3	0.00	33.3	-0.51 (0.44) [-1.44, 0.32]
			SoC	10	9 (90.0)	3.70 (7.35)	0.0	0.00	16.7	
		Week 20	Peg	19	17 (89.5)	1.96 (21.96)	-50.0	0.00	50.0	0.09 (0.45) [-0.78, 0.99]
			SoC	10	8 (80.0)	0.00 (15.43)	-33.3	0.00	16.7	
		Week 26	Peg	19	14 (73.7)	1.19 (17.86)	-50.0	0.00	33.3	0.22 (0.52) [-0.78, 1.27]
			SoC	10	6 (60.0)	-2.78 (16.39)	-33.3	0.00	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SIHD: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Nausea/Vomiting (censored)	Female	Week 4	Peg	16	15 (93.8)	-5.56 (13.61)	-50.0	0.00	0.0	-0.94 (0.51) [-2.06, -0.05]
			SoC	8	7 (87.5)	9.52 (18.90)	0.0	0.00	50.0	
		Week 8	Peg	16	14 (87.5)	-5.95 (14.03)	-50.0	0.00	0.0	-0.82 (0.51) [-1.94, 0.08]
			SoC	8	7 (87.5)	4.76 (8.13)	0.0	0.00	16.7	
		Week 12	Peg	16	14 (87.5)	-4.76 (13.76)	-50.0	0.00	0.0	-0.75 (0.51) [-1.84, 0.17]
			SoC	8	7 (87.5)	4.76 (8.13)	0.0	0.00	16.7	
		Week 16	Peg	16	14 (87.5)	-3.57 (14.88)	-50.0	0.00	16.7	-0.61 (0.50) [-1.68, 0.31]
			SoC	8	7 (87.5)	4.76 (8.13)	0.0	0.00	16.7	
		Week 20	Peg	16	15 (93.8)	-4.44 (14.73)	-50.0	0.00	16.7	-0.68 (0.50) [-1.74, 0.23]
			SoC	8	7 (87.5)	4.76 (8.13)	0.0	0.00	16.7	
		Week 26	Peg	16	13 (81.3)	-5.13 (14.25)	-50.0	0.00	0.0	-0.60 (0.54) [-1.75, 0.38]
			SoC	8	6 (75.0)	2.78 (6.80)	0.0	0.00	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SICB: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.0728

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SICB: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Latin America	Peg	Week 4	12	11 (91.7)	-9.09 (1.67)	(-12.87, -5.30)	-8.38 (4.40)	(-18.33, 1.57)	0.0892	
		Week 8	12	10 (83.3)	-10.93 (1.13)	(-13.55, -8.32)	-7.26 (2.92)	(-14.00, -0.52)	0.0379	*
		Week 12	12	11 (91.7)	-9.09 (1.67)	(-12.87, -5.30)	-8.38 (4.40)	(-18.33, 1.57)	0.0892	
		Week 16	12	10 (83.3)	-7.97 (2.63)	(-14.04, -1.91)	-6.18 (6.78)	(-21.81, 9.44)	0.3882	
		Week 20	12	10 (83.3)	-9.61 (1.16)	(-12.29, -6.93)	-7.65 (2.94)	(-14.43, -0.88)	0.0313	*
		Week 26	12	9 (75.0)	-10.70 (1.30)	(-13.77, -7.64)	-7.82 (3.14)	(-15.26, -0.39)	0.0417	*
	SoC	Week 4	2	2 (100.0)	-0.71 (4.04)	(-9.85, 8.44)				
		Week 8	2	2 (100.0)	-3.67 (2.64)	(-9.77, 2.42)				
		Week 12	2	2 (100.0)	-0.71 (4.04)	(-9.85, 8.44)				
		Week 16	2	2 (100.0)	-1.79 (6.13)	(-15.92, 12.33)				
		Week 20	2	2 (100.0)	-1.96 (2.66)	(-8.10, 4.19)				
	Week 26	2	2 (100.0)	-2.88 (2.84)	(-9.59, 3.83)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SICB: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Asia Pacific Peg	Peg	Week 4	23	20 (87.0)	-0.97 (2.24)	(-5.54, 3.60)	-4.86 (3.67)	(-12.36, 2.65)	0.1963	
		Week 8	23	19 (82.6)	-0.33 (1.44)	(-3.28, 2.63)	-3.36 (2.34)	(-8.15, 1.42)	0.1612	
		Week 12	23	20 (87.0)	1.51 (1.45)	(-1.45, 4.48)	-1.30 (2.38)	(-6.17, 3.56)	0.5876	
		Week 16	23	20 (87.0)	0.55 (2.08)	(-3.71, 4.80)	-2.25 (3.31)	(-9.00, 4.51)	0.5022	
		Week 20	23	22 (95.7)	4.16 (2.96)	(-1.88, 10.19)	3.60 (4.99)	(-6.59, 13.79)	0.4764	
	Week 26	23	18 (78.3)	3.65 (2.45)	(-1.41, 8.71)	5.24 (4.35)	(-3.74, 14.22)	0.2399		
	SoC	Week 4	16	13 (81.3)	3.88 (2.82)	(-1.89, 9.66)				
		Week 8	16	13 (81.3)	3.04 (1.76)	(-0.57, 6.65)				
		Week 12	16	13 (81.3)	2.82 (1.83)	(-0.93, 6.56)				
		Week 16	16	14 (87.5)	2.79 (2.51)	(-2.32, 7.91)				
Week 20		16	13 (81.3)	0.56 (3.92)	(-7.44, 8.55)					
Week 26	16	10 (62.5)	-1.59 (3.51)	(-8.84, 5.65)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SIHB: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Nausea/Vomiting (censored)	Latin America	Baseline	Peg	12	12 (100.0)	12.50 (18.97)	0.0	0.00	50.0	
			SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
		Week 4	Peg	12	11 (91.7)	1.52 (5.03)	0.0	0.00	16.7	
			SoC	2	2 (100.0)	8.33 (11.79)	0.0	8.33	16.7	
		Week 8	Peg	12	10 (83.3)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	2	2 (100.0)	8.33 (11.79)	0.0	8.33	16.7	
		Week 12	Peg	12	11 (91.7)	1.52 (5.03)	0.0	0.00	16.7	
			SoC	2	2 (100.0)	8.33 (11.79)	0.0	8.33	16.7	
		Week 16	Peg	12	10 (83.3)	3.33 (7.03)	0.0	0.00	16.7	
			SoC	2	2 (100.0)	8.33 (11.79)	0.0	8.33	16.7	
		Week 20	Peg	12	10 (83.3)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	2	2 (100.0)	8.33 (11.79)	0.0	8.33	16.7	
		Week 26	Peg	12	9 (75.0)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	2	2 (100.0)	8.33 (11.79)	0.0	8.33	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SIHB: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Nausea/Vomiting (censored)	Asia	Baseline	Peg	23	23 (100.0)	2.90 (8.18)	0.0	0.00	33.3	
			SoC	16	14 (87.5)	2.38 (8.91)	0.0	0.00	33.3	
	Pacific	Week 4	Peg	23	20 (87.0)	1.67 (5.13)	0.0	0.00	16.7	
			SoC	16	15 (93.8)	6.67 (13.80)	0.0	0.00	50.0	
		Week 8	Peg	23	19 (82.6)	2.63 (6.24)	0.0	0.00	16.7	
			SoC	16	14 (87.5)	5.95 (10.56)	0.0	0.00	33.3	
		Week 12	Peg	23	20 (87.0)	5.00 (15.39)	0.0	0.00	66.7	
			SoC	16	14 (87.5)	4.76 (10.19)	0.0	0.00	33.3	
		Week 16	Peg	23	20 (87.0)	3.33 (11.60)	0.0	0.00	50.0	
			SoC	16	15 (93.8)	5.56 (10.29)	0.0	0.00	33.3	
		Week 20	Peg	23	22 (95.7)	6.82 (15.99)	0.0	0.00	66.7	
			SoC	16	15 (93.8)	3.33 (6.90)	0.0	0.00	16.7	
		Week 26	Peg	23	18 (78.3)	6.48 (14.16)	0.0	0.00	50.0	
			SoC	16	11 (68.8)	1.52 (5.03)	0.0	0.00	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SIH8: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Nausea/Vomiting (censored)	Latin America	Week 4	Peg	12	11 (91.7)	-10.61 (17.12)	-50.0	0.00	0.0	-1.05 (0.89) [-3.06, 0.47]
			SoC	2	2 (100.0)	8.33 (11.79)	0.0	8.33	16.7	
		Week 8	Peg	12	10 (83.3)	-13.33 (20.49)	-50.0	0.00	0.0	-1.01 (0.91) [-3.07, 0.54]
			SoC	2	2 (100.0)	8.33 (11.79)	0.0	8.33	16.7	
		Week 12	Peg	12	11 (91.7)	-10.61 (17.12)	-50.0	0.00	0.0	-1.05 (0.89) [-3.06, 0.47]
			SoC	2	2 (100.0)	8.33 (11.79)	0.0	8.33	16.7	
		Week 16	Peg	12	10 (83.3)	-10.00 (19.56)	-50.0	0.00	16.7	-0.89 (0.90) [-2.91, 0.67]
			SoC	2	2 (100.0)	8.33 (11.79)	0.0	8.33	16.7	
		Week 20	Peg	12	10 (83.3)	-11.67 (20.86)	-50.0	0.00	0.0	-0.92 (0.90) [-2.94, 0.64]
			SoC	2	2 (100.0)	8.33 (11.79)	0.0	8.33	16.7	
		Week 26	Peg	12	9 (75.0)	-12.96 (21.70)	-50.0	0.00	0.0	-0.93 (0.93) [-3.05, 0.64]
			SoC	2	2 (100.0)	8.33 (11.79)	0.0	8.33	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SIHB: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Nausea/Vomiting (censored)	Asia Pacific	Week 4	Peg	23	20 (87.0)	-1.67 (9.21)	-33.3	0.00	16.7	-0.54 (0.38) [-1.32, 0.16]
			SoC	16	13 (81.3)	5.13 (15.79)	-16.7	0.00	50.0	
		Week 8	Peg	23	19 (82.6)	-0.88 (6.74)	-16.7	0.00	16.7	-0.66 (0.38) [-1.47, 0.05]
			SoC	16	13 (81.3)	3.85 (7.31)	0.0	0.00	16.7	
		Week 12	Peg	23	20 (87.0)	1.67 (7.45)	0.0	0.00	33.3	-0.12 (0.37) [-0.86, 0.59]
			SoC	16	13 (81.3)	2.56 (6.26)	0.0	0.00	16.7	
		Week 16	Peg	23	20 (87.0)	0.00 (10.81)	-33.3	0.00	33.3	-0.37 (0.36) [-1.11, 0.32]
			SoC	16	14 (87.5)	3.57 (7.10)	0.0	0.00	16.7	
		Week 20	Peg	23	22 (95.7)	3.79 (16.21)	-33.3	0.00	50.0	0.16 (0.36) [-0.54, 0.89]
			SoC	16	13 (81.3)	1.28 (12.66)	-33.3	0.00	16.7	
		Week 26	Peg	23	18 (78.3)	3.70 (9.14)	0.0	0.00	33.3	0.50 (0.42) [-0.28, 1.37]
			SoC	16	10 (62.5)	-1.67 (12.30)	-33.3	0.00	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SICA: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.5001

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SICA: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
< 4	Peg	Week 4	21	18 (85.7)	-7.33 (1.20)	(-9.84, -4.83)	-7.07 (2.65)	(-12.60, -1.54)	0.0148	*
		Week 8	21	17 (81.0)	-7.54 (1.34)	(-10.34, -4.74)	-6.50 (2.90)	(-12.57, -0.43)	0.0371	*
		Week 12	21	18 (85.7)	-3.42 (3.19)	(-10.09, 3.24)	-2.42 (7.06)	(-17.15, 12.30)	0.7352	
		Week 16	21	17 (81.0)	-6.44 (1.50)	(-9.57, -3.30)	-5.23 (3.05)	(-11.59, 1.13)	0.1019	
		Week 20	21	18 (85.7)	-3.30 (2.17)	(-7.84, 1.24)	-1.85 (4.78)	(-11.82, 8.12)	0.7033	
		Week 26	21	15 (71.4)	-4.04 (2.61)	(-9.60, 1.51)	-2.02 (6.52)	(-15.91, 11.87)	0.7608	
	SoC	Week 4	8	5 (62.5)	-0.26 (2.33)	(-5.11, 4.59)				
		Week 8	8	5 (62.5)	-1.04 (2.53)	(-6.32, 4.25)				
		Week 12	8	5 (62.5)	-1.00 (6.19)	(-13.93, 11.92)				
		Week 16	8	6 (75.0)	-1.21 (2.59)	(-6.61, 4.19)				
		Week 20	8	5 (62.5)	-1.45 (4.20)	(-10.22, 7.31)				
		Week 26	8	3 (37.5)	-2.02 (5.92)	(-14.65, 10.61)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SICA: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	0.75 (3.19)	(-5.90, 7.41)	-4.93 (4.86)	(-15.07, 5.21)	0.3224	
		Week 8	14	12 (85.7)	1.29 (1.74)	(-2.35, 4.93)	-2.15 (2.59)	(-7.58, 3.27)	0.4165	
		Week 12	14	13 (92.9)	-0.08 (1.30)	(-2.79, 2.63)	-3.51 (1.98)	(-7.64, 0.61)	0.0910	
		Week 16	14	13 (92.9)	4.12 (2.43)	(-0.96, 9.19)	1.13 (3.71)	(-6.60, 8.87)	0.7630	
		Week 20	14	14 (100.0)	4.51 (3.72)	(-3.22, 12.24)	4.15 (5.79)	(-7.89, 16.20)	0.4813	
		Week 26	14	12 (85.7)	2.35 (3.16)	(-4.30, 8.99)	3.62 (4.86)	(-6.59, 13.84)	0.4659	
	SoC	Week 4	10	10 (100.0)	5.69 (3.64)	(-1.91, 13.28)				
		Week 8	10	10 (100.0)	3.45 (1.91)	(-0.54, 7.44)				
		Week 12	10	10 (100.0)	3.44 (1.48)	(0.34, 6.53)				
		Week 16	10	10 (100.0)	2.98 (2.78)	(-2.81, 8.77)				
		Week 20	10	10 (100.0)	0.35 (4.41)	(-8.81, 9.52)				
		Week 26	10	9 (90.0)	-1.28 (3.66)	(-8.96, 6.41)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SIHA: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Nausea/Vomiting (censored)	< 4	Baseline	Peg	21	21 (100.0)	9.52 (16.31)	0.0	0.00	50.0	
			SoC	8	6 (75.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 4		Peg	21	18 (85.7)	0.93 (3.93)	0.0	0.00	16.7	
			SoC	8	7 (87.5)	4.76 (8.13)	0.0	0.00	16.7	
	Week 8		Peg	21	17 (81.0)	0.98 (4.04)	0.0	0.00	16.7	
			SoC	8	6 (75.0)	5.56 (8.61)	0.0	0.00	16.7	
	Week 12		Peg	21	18 (85.7)	5.56 (16.17)	0.0	0.00	66.7	
			SoC	8	6 (75.0)	2.78 (6.80)	0.0	0.00	16.7	
	Week 16		Peg	21	17 (81.0)	1.96 (5.54)	0.0	0.00	16.7	
			SoC	8	7 (87.5)	4.76 (8.13)	0.0	0.00	16.7	
	Week 20		Peg	21	18 (85.7)	3.70 (9.14)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	4.76 (8.13)	0.0	0.00	16.7	
	Week 26		Peg	21	15 (71.4)	4.44 (9.89)	0.0	0.00	33.3	
			SoC	8	4 (50.0)	4.17 (8.33)	0.0	0.00	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SIHA: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Nausea/Vomiting (censored)	>= 4	Baseline	Peg	14	14 (100.0)	1.19 (4.45)	0.0	0.00	16.7	
			SoC	10	10 (100.0)	3.33 (10.54)	0.0	0.00	33.3	
	Week 4	Peg	14	13 (92.9)	2.56 (6.26)	0.0	0.00	16.7		
		SoC	10	10 (100.0)	8.33 (16.20)	0.0	0.00	50.0		
	Week 8	Peg	14	12 (85.7)	2.78 (6.49)	0.0	0.00	16.7		
		SoC	10	10 (100.0)	6.67 (11.65)	0.0	0.00	33.3		
	Week 12	Peg	14	13 (92.9)	1.28 (4.62)	0.0	0.00	16.7		
		SoC	10	10 (100.0)	6.67 (11.65)	0.0	0.00	33.3		
	Week 16	Peg	14	13 (92.9)	5.13 (14.25)	0.0	0.00	50.0		
		SoC	10	10 (100.0)	6.67 (11.65)	0.0	0.00	33.3		
	Week 20	Peg	14	14 (100.0)	5.95 (18.03)	0.0	0.00	66.7		
		SoC	10	10 (100.0)	3.33 (7.03)	0.0	0.00	16.7		
	Week 26	Peg	14	12 (85.7)	4.17 (14.43)	0.0	0.00	50.0		
		SoC	10	9 (90.0)	1.85 (5.56)	0.0	0.00	16.7		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SIHA: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in QLQ-C30 Nausea/Vomiting (censored)	Week 4	Peg	21	18 (85.7)	-9.26 (15.36)	-50.0	0.00	0.0	-1.07 (0.56) [-2.29, -0.08]
		SoC	8	5 (62.5)	6.67 (9.13)	0.0	0.00	16.7	
	Week 8	Peg	21	17 (81.0)	-9.80 (16.73)	-50.0	0.00	0.0	-1.02 (0.57) [-2.26, -0.02]
		SoC	8	5 (62.5)	6.67 (9.13)	0.0	0.00	16.7	
	Week 12	Peg	21	18 (85.7)	-4.63 (16.96)	-50.0	0.00	33.3	-0.49 (0.54) [-1.61, 0.52]
		SoC	8	5 (62.5)	3.33 (7.45)	0.0	0.00	16.7	
	Week 16	Peg	21	17 (81.0)	-8.82 (15.72)	-50.0	0.00	0.0	-0.97 (0.53) [-2.11, -0.04]
		SoC	8	6 (75.0)	5.56 (8.61)	0.0	0.00	16.7	
	Week 20	Peg	21	18 (85.7)	-5.56 (21.39)	-50.0	0.00	33.3	-0.60 (0.54) [-1.74, 0.40]
		SoC	8	5 (62.5)	6.67 (9.13)	0.0	0.00	16.7	
	Week 26	Peg	21	15 (71.4)	-5.56 (19.59)	-50.0	0.00	16.7	-0.57 (0.69) [-2.01, 0.71]
		SoC	8	3 (37.5)	5.56 (9.62)	0.0	0.00	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QVWC_SIHA: Change from baseline in QLQ-C30 Nausea/Vomiting (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in QLQ-C30 Nausea/Vomiting (censored)	Week 4	Peg	14	13 (92.9)	1.28 (4.62)	0.0	0.00	16.7	-0.30 (0.45) [-1.21, 0.55]
		SoC	10	10 (100.0)	5.00 (17.66)	-16.7	0.00	50.0	
	Week 8	Peg	14	12 (85.7)	1.39 (4.81)	0.0	0.00	16.7	-0.32 (0.45) [-1.25, 0.55]
		SoC	10	10 (100.0)	3.33 (7.03)	0.0	0.00	16.7	
	Week 12	Peg	14	13 (92.9)	0.00 (0.00)	0.0	0.00	0.0	-0.70 (0.46) [-1.68, 0.13]
		SoC	10	10 (100.0)	3.33 (7.03)	0.0	0.00	16.7	
	Week 16	Peg	14	13 (92.9)	3.85 (9.99)	0.0	0.00	33.3	0.06 (0.44) [-0.81, 0.94]
		SoC	10	10 (100.0)	3.33 (7.03)	0.0	0.00	16.7	
	Week 20	Peg	14	14 (100.0)	4.76 (13.76)	0.0	0.00	50.0	0.34 (0.44) [-0.49, 1.24]
		SoC	10	10 (100.0)	0.00 (13.61)	-33.3	0.00	16.7	
	Week 26	Peg	14	12 (85.7)	2.78 (9.62)	0.0	0.00	33.3	0.40 (0.47) [-0.49, 1.38]
		SoC	10	9 (90.0)	-1.85 (13.03)	-33.3	0.00	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SICC: Change from baseline in QLQ-C30 Dyspnoea (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA				
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA	
Age	Peg	Overall									0.7463

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SICC: Change from baseline in QLQ-C30 Dyspnoea (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 60	Peg	Week 4	29	26 (89.7)	-10.02 (3.13)	(-16.40, -3.65)	-9.51 (5.86)	(-21.42, 2.40)	0.1139	
		Week 8	29	24 (82.8)	-3.11 (4.50)	(-12.28, 6.07)	-12.70 (8.22)	(-29.47, 4.06)	0.1324	
		Week 12	29	26 (89.7)	-5.37 (3.55)	(-12.60, 1.85)	-8.76 (6.67)	(-22.32, 4.81)	0.1980	
		Week 16	29	25 (86.2)	-4.52 (3.97)	(-12.61, 3.56)	-5.72 (7.14)	(-20.25, 8.82)	0.4292	
		Week 20	29	27 (93.1)	-6.11 (3.49)	(-13.21, 0.99)	-8.98 (6.63)	(-22.46, 4.50)	0.1846	
		Week 26	29	23 (79.3)	-7.10 (3.88)	(-15.04, 0.84)	-13.41 (7.36)	(-28.47, 1.65)	0.0789	
	SoC	Week 4	13	11 (84.6)	-0.51 (4.85)	(-10.38, 9.36)				
		Week 8	13	11 (84.6)	9.59 (6.70)	(-4.08, 23.27)				
		Week 12	13	11 (84.6)	3.39 (5.51)	(-7.83, 14.60)				
		Week 16	13	12 (92.3)	1.20 (5.79)	(-10.59, 12.98)				
		Week 20	13	11 (84.6)	2.87 (5.54)	(-8.39, 14.14)				
		Week 26	13	10 (76.9)	6.31 (6.07)	(-6.10, 18.73)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SICC: Change from baseline in QLQ-C30 Dyspnoea (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	-13.89 (14.92)	(-52.24, 24.46)	-38.89 (23.78)	(-100.03, 22.25)	0.1630	
		Week 8	6	5 (83.3)	-13.33 (9.60)	(-38.00, 11.34)	-20.00 (15.30)	(-59.33, 19.33)	0.2480	
		Week 12	6	5 (83.3)	-21.44 (8.95)	(-44.45, 1.57)	-31.33 (14.27)	(-68.02, 5.35)	0.0795	
		Week 16	6	5 (83.3)	-18.65 (12.29)	(-50.25, 12.94)	-25.32 (19.41)	(-75.22, 24.57)	0.2488	
		Week 20	6	5 (83.3)	-11.11 (11.89)	(-41.67, 19.44)	-15.15 (18.77)	(-63.40, 33.10)	0.4562	
		Week 26	6	4 (66.7)	-22.69 (7.64)	(-55.57, 10.20)	-16.67 (15.21)	(-82.13, 48.80)	0.3876	
	SoC	Week 4	5	4 (80.0)	25.00 (17.56)	(-20.15, 70.15)				
		Week 8	5	4 (80.0)	6.67 (11.30)	(-22.38, 35.71)				
		Week 12	5	4 (80.0)	9.89 (10.54)	(-17.20, 36.98)				
		Week 16	5	4 (80.0)	6.67 (14.40)	(-30.33, 43.68)				
		Week 20	5	4 (80.0)	4.04 (13.92)	(-31.75, 39.83)				
		Week 26	5	2 (40.0)	-6.02 (13.20)	(-62.80, 50.76)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SIHC: Change from baseline in QLQ-C30 Dyspnoea (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Dyspnoea (censored)	< 60	Baseline	Peg	29	29 (100.0)	14.94 (19.08)	0.0	0.00	66.7	
			SoC	13	12 (92.3)	22.22 (16.41)	0.0	33.33	33.3	
		Week 4	Peg	29	26 (89.7)	6.41 (16.38)	0.0	0.00	66.7	
			SoC	13	12 (92.3)	16.67 (17.41)	0.0	16.67	33.3	
		Week 8	Peg	29	24 (82.8)	12.50 (23.70)	0.0	0.00	66.7	
			SoC	13	11 (84.6)	27.27 (20.10)	0.0	33.33	66.7	
		Week 12	Peg	29	26 (89.7)	10.26 (18.30)	0.0	0.00	66.7	
			SoC	13	11 (84.6)	21.21 (22.47)	0.0	33.33	66.7	
		Week 16	Peg	29	25 (86.2)	10.67 (20.91)	0.0	0.00	66.7	
			SoC	13	12 (92.3)	19.44 (17.16)	0.0	33.33	33.3	
		Week 20	Peg	29	27 (93.1)	9.88 (20.29)	0.0	0.00	66.7	
			SoC	13	12 (92.3)	19.44 (17.16)	0.0	33.33	33.3	
		Week 26	Peg	29	23 (79.3)	10.14 (18.63)	0.0	0.00	66.7	
			SoC	13	10 (76.9)	26.67 (21.08)	0.0	33.33	66.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SIHC: Change from baseline in QLQ-C30 Dyspnoea (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Dyspnoea (censored)	>= 60	Baseline	Peg	6	6 (100.0)	27.78 (32.77)	0.0	16.67	66.7	
			SoC	5	4 (80.0)	25.00 (16.67)	0.0	33.33	33.3	
		Week 4	Peg	6	5 (83.3)	6.67 (14.91)	0.0	0.00	33.3	
			SoC	5	5 (100.0)	46.67 (38.01)	0.0	33.33	100.0	
		Week 8	Peg	6	5 (83.3)	6.67 (14.91)	0.0	0.00	33.3	
			SoC	5	5 (100.0)	26.67 (27.89)	0.0	33.33	66.7	
		Week 12	Peg	6	5 (83.3)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	5	5 (100.0)	26.67 (27.89)	0.0	33.33	66.7	
		Week 16	Peg	6	5 (83.3)	13.33 (29.81)	0.0	0.00	66.7	
			SoC	5	5 (100.0)	26.67 (27.89)	0.0	33.33	66.7	
		Week 20	Peg	6	5 (83.3)	20.00 (29.81)	0.0	0.00	66.7	
			SoC	5	5 (100.0)	26.67 (27.89)	0.0	33.33	66.7	
		Week 26	Peg	6	4 (66.7)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	5	3 (60.0)	11.11 (19.25)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SIHC: Change from baseline in QLQ-C30 Dyspnoea (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in QLQ-C30 Dyspnoea (censored)	Week 4	Peg	29	26 (89.7)	-8.97 (20.13)	-33.3	0.00	33.3	-0.30 (0.37) [-1.05, 0.42]
		SoC	13	11 (84.6)	-3.03 (17.98)	-33.3	0.00	33.3	
	Week 8	Peg	29	24 (82.8)	-1.39 (25.02)	-33.3	0.00	66.7	-0.31 (0.38) [-1.07, 0.42]
		SoC	13	11 (84.6)	6.06 (20.10)	-33.3	0.00	33.3	
	Week 12	Peg	29	26 (89.7)	-3.85 (19.61)	-33.3	0.00	33.3	-0.19 (0.37) [-0.93, 0.53]
		SoC	13	11 (84.6)	0.00 (21.08)	-33.3	0.00	33.3	
	Week 16	Peg	29	25 (86.2)	-2.67 (23.41)	-33.3	0.00	33.3	0.00 (0.36) [-0.71, 0.72]
		SoC	13	12 (92.3)	-2.78 (22.29)	-33.3	0.00	33.3	
	Week 20	Peg	29	27 (93.1)	-4.94 (22.08)	-33.3	0.00	33.3	-0.24 (0.37) [-0.98, 0.48]
		SoC	13	11 (84.6)	0.00 (14.91)	-33.3	0.00	33.3	
	Week 26	Peg	29	23 (79.3)	-5.80 (21.68)	-33.3	0.00	33.3	-0.43 (0.40) [-1.24, 0.32]
		SoC	13	10 (76.9)	3.33 (18.92)	-33.3	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SIHC: Change from baseline in QLQ-C30 Dyspnoea (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in QLQ-C30 Dyspnoea (censored)	Week 4	Peg	6	5 (83.3)	-13.33 (29.81)	-66.7	0.00	0.0	-1.11 (0.89) [-3.29, 0.22]
		SoC	5	4 (80.0)	25.00 (31.91)	0.0	16.67	66.7	
	Week 8	Peg	6	5 (83.3)	-13.33 (29.81)	-66.7	0.00	0.0	-0.77 (0.84) [-2.73, 0.60]
		SoC	5	4 (80.0)	8.33 (16.67)	0.0	0.00	33.3	
	Week 12	Peg	6	5 (83.3)	-20.00 (29.81)	-66.7	0.00	0.0	-1.01 (0.88) [-3.12, 0.33]
		SoC	5	4 (80.0)	8.33 (16.67)	0.0	0.00	33.3	
	Week 16	Peg	6	5 (83.3)	-20.00 (29.81)	-66.7	0.00	0.0	-1.01 (0.88) [-3.12, 0.33]
		SoC	5	4 (80.0)	8.33 (16.67)	0.0	0.00	33.3	
	Week 20	Peg	6	5 (83.3)	-13.33 (29.81)	-66.7	0.00	0.0	-0.77 (0.84) [-2.73, 0.60]
		SoC	5	4 (80.0)	8.33 (16.67)	0.0	0.00	33.3	
	Week 26	Peg	6	4 (66.7)	-25.00 (31.91)	-66.7	-16.67	0.0	-0.72 (1.31) [-3.84, 1.22]
		SoC	5	2 (40.0)	0.00 (0.00)	0.0	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SICD: Change from baseline in QLQ-C30 Dyspnoea (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.5205

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SICD: Change from baseline in QLQ-C30 Dyspnoea (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Male	Peg	Week 4	19	16 (84.2)	-7.20 (4.57)	(-16.73, 2.32)	-14.16 (7.98)	(-30.80, 2.47)	0.0910	
		Week 8	19	15 (78.9)	-5.61 (5.03)	(-16.13, 4.91)	-16.13 (8.68)	(-34.29, 2.03)	0.0786	
		Week 12	19	17 (89.5)	-6.85 (3.84)	(-14.82, 1.13)	-12.78 (6.90)	(-27.13, 1.57)	0.0782	
		Week 16	19	16 (84.2)	-4.94 (5.02)	(-15.38, 5.49)	-3.35 (8.50)	(-21.02, 14.33)	0.6977	
		Week 20	19	17 (89.5)	-2.17 (4.40)	(-11.32, 6.99)	-3.67 (7.82)	(-19.93, 12.58)	0.6434	
		Week 26	19	14 (73.7)	-8.41 (5.32)	(-19.69, 2.87)	-16.93 (9.88)	(-37.87, 4.01)	0.1058	
	SoC	Week 4	10	8 (80.0)	6.96 (6.51)	(-6.63, 20.54)				
		Week 8	10	8 (80.0)	10.52 (6.99)	(-4.10, 25.14)				
		Week 12	10	8 (80.0)	5.93 (5.67)	(-5.86, 17.72)				
		Week 16	10	9 (90.0)	-1.60 (6.82)	(-15.77, 12.58)				
		Week 20	10	8 (80.0)	1.51 (6.47)	(-11.94, 14.95)				
		Week 26	10	6 (60.0)	8.52 (8.21)	(-8.88, 25.92)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SICD: Change from baseline in QLQ-C30 Dyspnoea (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Female	Peg	Week 4	16	15 (93.8)	-13.68 (5.82)	(-25.92, -1.45)	-17.73 (12.30)	(-43.57, 8.11)	0.1666	
		Week 8	16	14 (87.5)	-3.40 (6.59)	(-17.29, 10.50)	-9.67 (13.87)	(-38.94, 19.60)	0.4953	
		Week 12	16	14 (87.5)	-8.73 (5.56)	(-20.45, 3.00)	-12.04 (11.71)	(-36.74, 12.66)	0.3182	
		Week 16	16	14 (87.5)	-8.87 (5.44)	(-20.34, 2.59)	-17.26 (11.45)	(-41.42, 6.90)	0.1501	
		Week 20	16	15 (93.8)	-12.81 (4.53)	(-22.32, -3.30)	-23.41 (9.56)	(-43.49, -3.32)	0.0248	*
		Week 26	16	13 (81.3)	-10.30 (4.34)	(-19.55, -1.06)	-9.70 (9.23)	(-29.37, 9.96)	0.3097	
	SoC	Week 4	8	7 (87.5)	4.04 (10.18)	(-17.34, 25.43)				
		Week 8	8	7 (87.5)	6.27 (11.11)	(-17.17, 29.71)				
		Week 12	8	7 (87.5)	3.31 (9.38)	(-16.47, 23.10)				
		Week 16	8	7 (87.5)	8.39 (9.17)	(-10.96, 27.74)				
		Week 20	8	7 (87.5)	10.60 (7.91)	(-6.02, 27.23)				
		Week 26	8	6 (75.0)	-0.60 (7.58)	(-16.77, 15.56)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SIHD: Change from baseline in QLQ-C30 Dyspnoea (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Dyspnoea (censored)	Male	Baseline	Peg	19	19 (100.0)	17.54 (23.22)	0.0	0.00	66.7	
			SoC	10	9 (90.0)	25.93 (14.70)	0.0	33.33	33.3	
		Week 4	Peg	19	16 (84.2)	10.42 (20.07)	0.0	0.00	66.7	
			SoC	10	9 (90.0)	29.63 (20.03)	0.0	33.33	66.7	
		Week 8	Peg	19	15 (78.9)	11.11 (24.12)	0.0	0.00	66.7	
			SoC	10	9 (90.0)	29.63 (20.03)	0.0	33.33	66.7	
		Week 12	Peg	19	17 (89.5)	9.80 (19.60)	0.0	0.00	66.7	
			SoC	10	9 (90.0)	25.93 (22.22)	0.0	33.33	66.7	
		Week 16	Peg	19	16 (84.2)	14.58 (27.13)	0.0	0.00	66.7	
			SoC	10	10 (100.0)	20.00 (17.21)	0.0	33.33	33.3	
		Week 20	Peg	19	17 (89.5)	17.65 (26.66)	0.0	0.00	66.7	
			SoC	10	9 (90.0)	22.22 (16.67)	0.0	33.33	33.3	
		Week 26	Peg	19	14 (73.7)	11.90 (21.11)	0.0	0.00	66.7	
			SoC	10	7 (70.0)	28.57 (23.00)	0.0	33.33	66.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SIHD: Change from baseline in QLQ-C30 Dyspnoea (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Dyspnoea (censored)	Female	Baseline	Peg	16	16 (100.0)	16.67 (21.08)	0.0	0.00	66.7	
			SoC	8	7 (87.5)	19.05 (17.82)	0.0	33.33	33.3	
		Week 4	Peg	16	15 (93.8)	2.22 (8.61)	0.0	0.00	33.3	
			SoC	8	8 (100.0)	20.83 (35.36)	0.0	0.00	100.0	
		Week 8	Peg	16	14 (87.5)	11.90 (21.11)	0.0	0.00	66.7	
			SoC	8	7 (87.5)	23.81 (25.20)	0.0	33.33	66.7	
		Week 12	Peg	16	14 (87.5)	7.14 (14.19)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	19.05 (26.23)	0.0	0.00	66.7	
		Week 16	Peg	16	14 (87.5)	7.14 (14.19)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	23.81 (25.20)	0.0	33.33	66.7	
		Week 20	Peg	16	15 (93.8)	4.44 (11.73)	0.0	0.00	33.3	
			SoC	8	8 (100.0)	20.83 (24.80)	0.0	16.67	66.7	
		Week 26	Peg	16	13 (81.3)	5.13 (12.52)	0.0	0.00	33.3	
			SoC	8	6 (75.0)	16.67 (18.26)	0.0	16.67	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SIHD: Change from baseline in QLQ-C30 Dyspnoea (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Dyspnoea (censored)	Male	Week 4	Peg	19	16 (84.2)	-6.25 (18.13)	-33.3	0.00	33.3	-0.52 (0.46) [-1.49, 0.33]
			SoC	10	8 (80.0)	4.17 (21.36)	-33.3	0.00	33.3	
		Week 8	Peg	19	15 (78.9)	-4.44 (17.21)	-33.3	0.00	33.3	-0.63 (0.47) [-1.63, 0.23]
			SoC	10	8 (80.0)	8.33 (23.57)	-33.3	0.00	33.3	
		Week 12	Peg	19	17 (89.5)	-5.88 (13.10)	-33.3	0.00	0.0	-0.60 (0.46) [-1.57, 0.24]
			SoC	10	8 (80.0)	4.17 (21.36)	-33.3	0.00	33.3	
		Week 16	Peg	19	16 (84.2)	-4.17 (20.64)	-33.3	0.00	33.3	-0.02 (0.44) [-0.89, 0.84]
			SoC	10	9 (90.0)	-3.70 (20.03)	-33.3	0.00	33.3	
		Week 20	Peg	19	17 (89.5)	-1.96 (18.52)	-33.3	0.00	33.3	-0.10 (0.45) [-1.00, 0.77]
			SoC	10	8 (80.0)	0.00 (17.82)	-33.3	0.00	33.3	
		Week 26	Peg	19	14 (73.7)	-7.14 (19.30)	-33.3	0.00	33.3	-0.58 (0.53) [-1.70, 0.39]
			SoC	10	6 (60.0)	5.56 (25.09)	-33.3	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SIHD: Change from baseline in QLQ-C30 Dyspnoea (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Dyspnoea (censored)	Female	Week 4	Peg	16	15 (93.8)	-13.33 (24.56)	-66.7	0.00	33.3	-0.66 (0.50) [-1.72, 0.24]
			SoC	8	7 (87.5)	4.76 (29.99)	-33.3	0.00	66.7	
		Week 8	Peg	16	14 (87.5)	-2.38 (33.24)	-66.7	0.00	66.7	-0.24 (0.49) [-1.24, 0.70]
			SoC	8	7 (87.5)	4.76 (12.60)	0.0	0.00	33.3	
		Week 12	Peg	16	14 (87.5)	-7.14 (29.75)	-66.7	0.00	33.3	-0.26 (0.49) [-1.26, 0.69]
			SoC	8	7 (87.5)	0.00 (19.25)	-33.3	0.00	33.3	
		Week 16	Peg	16	14 (87.5)	-7.14 (29.75)	-66.7	0.00	33.3	-0.41 (0.49) [-1.44, 0.52]
			SoC	8	7 (87.5)	4.76 (23.00)	-33.3	0.00	33.3	
		Week 20	Peg	16	15 (93.8)	-11.11 (27.22)	-66.7	0.00	33.3	-0.64 (0.50) [-1.70, 0.26]
			SoC	8	7 (87.5)	4.76 (12.60)	0.0	0.00	33.3	
		Week 26	Peg	16	13 (81.3)	-10.26 (28.50)	-66.7	0.00	33.3	-0.41 (0.53) [-1.52, 0.59]
			SoC	8	6 (75.0)	0.00 (0.00)	0.0	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SICB: Change from baseline in QLQ-C30 Dyspnoea (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.8986

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SICB: Change from baseline in QLQ-C30 Dyspnoea (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Latin America	Peg	Week 4	12	11 (91.7)	-17.74 (2.13)	(-22.56, -12.91)	-16.37 (5.42)	(-28.64, -4.11)	0.0145	*
		Week 8	12	10 (83.3)	-16.67 (2.34)	(-22.05, -11.28)	-16.67 (5.72)	(-29.86, -3.47)	0.0195	*
		Week 12	12	11 (91.7)	-17.74 (2.13)	(-22.56, -12.91)	-16.37 (5.42)	(-28.64, -4.11)	0.0145	*
		Week 16	12	10 (83.3)	-16.67 (2.34)	(-22.05, -11.28)	-16.67 (5.72)	(-29.86, -3.47)	0.0195	*
		Week 20	12	10 (83.3)	-19.41 (2.36)	(-24.86, -13.96)	-16.47 (5.80)	(-29.83, -3.10)	0.0218	*
		Week 26	12	9 (75.0)	-17.46 (4.96)	(-29.20, -5.73)	-12.76 (11.64)	(-40.28, 14.77)	0.3094	
	SoC	Week 4	2	2 (100.0)	-1.37 (4.99)	(-12.64, 9.91)				
		Week 8	2	2 (100.0)	-0.00 (5.22)	(-12.04, 12.04)				
		Week 12	2	2 (100.0)	-1.37 (4.99)	(-12.64, 9.91)				
		Week 16	2	2 (100.0)	-0.00 (5.22)	(-12.04, 12.04)				
		Week 20	2	2 (100.0)	-2.95 (5.29)	(-15.14, 9.25)				
		Week 26	2	2 (100.0)	-4.71 (10.52)	(-29.59, 20.18)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SICB: Change from baseline in QLQ-C30 Dyspnoea (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Asia Pacific Peg	Peg	Week 4	23	20 (87.0)	-6.05 (4.75)	(-15.77, 3.67)	-12.82 (7.89)	(-28.96, 3.32)	0.1152	
		Week 8	23	19 (82.6)	1.83 (4.98)	(-8.37, 12.03)	-8.32 (8.19)	(-25.09, 8.46)	0.3185	
		Week 12	23	20 (87.0)	-1.92 (3.96)	(-10.01, 6.17)	-7.75 (6.63)	(-21.30, 5.80)	0.2516	
		Week 16	23	20 (87.0)	-1.05 (4.79)	(-10.84, 8.73)	-2.56 (7.69)	(-18.26, 13.15)	0.7417	
		Week 20	23	22 (95.7)	-0.86 (3.73)	(-8.46, 6.74)	-5.01 (6.34)	(-17.95, 7.92)	0.4354	
	Week 26	23	18 (78.3)	-5.22 (4.29)	(-14.09, 3.64)	-11.78 (7.80)	(-27.87, 4.31)	0.1440		
	SoC	Week 4	16	13 (81.3)	6.77 (6.03)	(-5.56, 19.09)				
		Week 8	16	13 (81.3)	10.15 (6.11)	(-2.38, 22.67)				
		Week 12	16	13 (81.3)	5.83 (5.03)	(-4.46, 16.12)				
		Week 16	16	14 (87.5)	1.50 (5.78)	(-10.30, 13.31)				
Week 20		16	13 (81.3)	4.15 (4.95)	(-5.95, 14.26)					
Week 26	16	10 (62.5)	6.55 (6.23)	(-6.31, 19.42)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SIHB: Change from baseline in QLQ-C30 Dyspnoea (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Dyspnoea (censored)	Latin America	Baseline	Peg	12	12 (100.0)	19.44 (22.29)	0.0	16.67	66.7	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 4	Peg	12	11 (91.7)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 8	Peg	12	10 (83.3)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 12	Peg	12	11 (91.7)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 16	Peg	12	10 (83.3)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 20	Peg	12	10 (83.3)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 26	Peg	12	9 (75.0)	3.70 (11.11)	0.0	0.00	33.3	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SIHB: Change from baseline in QLQ-C30 Dyspnoea (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Dyspnoea (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	15.94 (22.18)	0.0	0.00	66.7	
			SoC	16	14 (87.5)	23.81 (15.63)	0.0	33.33	33.3	
		Week 4	Peg	23	20 (87.0)	10.00 (19.04)	0.0	0.00	66.7	
	SoC		16	15 (93.8)	26.67 (28.73)	0.0	33.33	100.0		
		Week 8	Peg	23	19 (82.6)	17.54 (25.74)	0.0	0.00	66.7	
	SoC		16	14 (87.5)	28.57 (22.10)	0.0	33.33	66.7		
		Week 12	Peg	23	20 (87.0)	13.33 (19.94)	0.0	0.00	66.7	
	SoC		16	14 (87.5)	23.81 (24.21)	0.0	33.33	66.7		
		Week 16	Peg	23	20 (87.0)	16.67 (25.36)	0.0	0.00	66.7	
	SoC		16	15 (93.8)	22.22 (20.57)	0.0	33.33	66.7		
		Week 20	Peg	23	22 (95.7)	16.67 (24.67)	0.0	0.00	66.7	
	SoC		16	15 (93.8)	22.22 (20.57)	0.0	33.33	66.7		
		Week 26	Peg	23	18 (78.3)	11.11 (19.80)	0.0	0.00	66.7	
	SoC		16	11 (68.8)	24.24 (21.56)	0.0	33.33	66.7		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SIHB: Change from baseline in QLQ-C30 Dyspnoea (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Dyspnoea (censored)	Latin America	Week 4	Peg	12	11 (91.7)	-18.18 (22.92)	-66.7	0.00	0.0	-0.65 (0.87) [-2.52, 0.92]
			SoC	2	2 (100.0)	0.00 (47.14)	-33.3	0.00	33.3	
		Week 8	Peg	12	10 (83.3)	-16.67 (23.57)	-66.7	0.00	0.0	-0.57 (0.88) [-2.47, 1.03]
			SoC	2	2 (100.0)	0.00 (47.14)	-33.3	0.00	33.3	
		Week 12	Peg	12	11 (91.7)	-18.18 (22.92)	-66.7	0.00	0.0	-0.65 (0.87) [-2.52, 0.92]
			SoC	2	2 (100.0)	0.00 (47.14)	-33.3	0.00	33.3	
		Week 16	Peg	12	10 (83.3)	-16.67 (23.57)	-66.7	0.00	0.0	-0.57 (0.88) [-2.47, 1.03]
			SoC	2	2 (100.0)	0.00 (47.14)	-33.3	0.00	33.3	
		Week 20	Peg	12	10 (83.3)	-20.00 (23.31)	-66.7	-16.67	0.0	-0.69 (0.89) [-2.63, 0.90]
			SoC	2	2 (100.0)	0.00 (47.14)	-33.3	0.00	33.3	
		Week 26	Peg	12	9 (75.0)	-18.52 (24.22)	-66.7	0.00	0.0	-0.61 (0.91) [-2.59, 1.01]
			SoC	2	2 (100.0)	0.00 (47.14)	-33.3	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SIHB: Change from baseline in QLQ-C30 Dyspnoea (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Dyspnoea (censored)	Asia	Week 4	Peg	23	20 (87.0)	-5.00 (19.57)	-33.3	0.00	33.3	-0.47 (0.37) [-1.24, 0.23]
			SoC	16	13 (81.3)	5.13 (22.96)	-33.3	0.00	66.7	
	Pacific	Week 8	Peg	23	19 (82.6)	3.51 (24.58)	-33.3	0.00	66.7	-0.19 (0.37) [-0.94, 0.53]
			SoC	16	13 (81.3)	7.69 (14.62)	0.0	0.00	33.3	
		Week 12	Peg	23	20 (87.0)	0.00 (18.73)	-33.3	0.00	33.3	-0.14 (0.37) [-0.88, 0.58]
			SoC	16	13 (81.3)	2.56 (16.45)	-33.3	0.00	33.3	
		Week 16	Peg	23	20 (87.0)	0.00 (24.18)	-33.3	0.00	33.3	NE (NE) [NE, NE]
			SoC	16	14 (87.5)	0.00 (18.49)	-33.3	0.00	33.3	
		Week 20	Peg	23	22 (95.7)	0.00 (20.57)	-33.3	0.00	33.3	-0.14 (0.36) [-0.87, 0.56]
			SoC	16	13 (81.3)	2.56 (9.25)	0.0	0.00	33.3	
		Week 26	Peg	23	18 (78.3)	-3.70 (22.55)	-33.3	0.00	33.3	-0.35 (0.41) [-1.20, 0.43]
			SoC	16	10 (62.5)	3.33 (10.54)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SICA: Change from baseline in QLQ-C30 Dyspnoea (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.5790

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SICA: Change from baseline in QLQ-C30 Dyspnoea (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 4	21	18 (85.7)	-12.51 (4.37)	(-21.62, -3.40)	-17.56 (9.50)	(-37.39, 2.26)	0.0794	
		Week 8	21	17 (81.0)	-3.76 (5.96)	(-16.24, 8.71)	-9.90 (12.72)	(-36.53, 16.74)	0.4463	
		Week 12	21	18 (85.7)	-6.84 (4.75)	(-16.75, 3.06)	-11.47 (10.33)	(-33.03, 10.09)	0.2802	
		Week 16	21	17 (81.0)	-7.16 (5.09)	(-17.77, 3.45)	0.33 (10.05)	(-20.63, 21.28)	0.9743	
		Week 20	21	18 (85.7)	-6.34 (4.86)	(-16.49, 3.80)	-2.51 (10.48)	(-24.37, 19.36)	0.8133	
		Week 26	21	15 (71.4)	-12.87 (4.79)	(-23.06, -2.67)	-21.64 (11.97)	(-47.16, 3.88)	0.0908	
	SoC	Week 4	8	5 (62.5)	5.05 (8.37)	(-12.42, 22.52)				
		Week 8	8	5 (62.5)	6.13 (11.13)	(-17.16, 29.42)				
		Week 12	8	5 (62.5)	4.63 (9.11)	(-14.37, 23.62)				
		Week 16	8	6 (75.0)	-7.49 (8.61)	(-25.45, 10.47)				
		Week 20	8	5 (62.5)	-3.83 (9.26)	(-23.15, 15.48)				
		Week 26	8	3 (37.5)	8.77 (10.88)	(-14.43, 31.97)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SICA: Change from baseline in QLQ-C30 Dyspnoea (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	-8.70 (6.05)	(-21.32, 3.93)	-16.67 (9.23)	(-35.92, 2.58)	0.0860	
		Week 8	14	12 (85.7)	-6.58 (4.99)	(-17.04, 3.87)	-17.82 (7.48)	(-33.46, -2.17)	0.0278	*
		Week 12	14	13 (92.9)	-9.66 (4.69)	(-19.44, 0.12)	-15.54 (7.20)	(-30.57, -0.51)	0.0433	*
		Week 16	14	13 (92.9)	-7.08 (5.58)	(-18.73, 4.56)	-16.29 (8.58)	(-34.18, 1.60)	0.0720	
		Week 20	14	14 (100.0)	-7.92 (4.51)	(-17.31, 1.46)	-15.69 (7.05)	(-30.35, -1.02)	0.0373	*
	Week 26	14	12 (85.7)	-6.61 (5.15)	(-17.43, 4.21)	-11.71 (7.91)	(-28.33, 4.91)	0.1560		
	SoC	Week 4	10	10 (100.0)	7.97 (6.91)	(-6.44, 22.38)				
		Week 8	10	10 (100.0)	11.23 (5.48)	(-0.24, 22.70)				
		Week 12	10	10 (100.0)	5.89 (5.36)	(-5.30, 17.07)				
		Week 16	10	10 (100.0)	9.21 (6.38)	(-4.11, 22.53)				
		Week 20	10	10 (100.0)	7.76 (5.36)	(-3.37, 18.90)				
Week 26	10	9 (90.0)	5.11 (5.95)	(-7.41, 17.62)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SIHA: Change from baseline in QLQ-C30 Dyspnoea (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Dyspnoea (censored)	< 4	Baseline	Peg	21	21 (100.0)	19.05 (24.88)	0.0	0.00	66.7	
			SoC	8	6 (75.0)	27.78 (13.61)	0.0	33.33	33.3	
		Week 4	Peg	21	18 (85.7)	5.56 (17.15)	0.0	0.00	66.7	
			SoC	8	7 (87.5)	23.81 (25.20)	0.0	33.33	66.7	
		Week 8	Peg	21	17 (81.0)	13.73 (26.51)	0.0	0.00	66.7	
			SoC	8	6 (75.0)	22.22 (17.21)	0.0	33.33	33.3	
		Week 12	Peg	21	18 (85.7)	11.11 (19.80)	0.0	0.00	66.7	
			SoC	8	6 (75.0)	22.22 (27.22)	0.0	16.67	66.7	
		Week 16	Peg	21	17 (81.0)	13.73 (23.74)	0.0	0.00	66.7	
			SoC	8	7 (87.5)	14.29 (17.82)	0.0	0.00	33.3	
		Week 20	Peg	21	18 (85.7)	14.81 (23.49)	0.0	0.00	66.7	
			SoC	8	7 (87.5)	14.29 (17.82)	0.0	0.00	33.3	
		Week 26	Peg	21	15 (71.4)	8.89 (15.26)	0.0	0.00	33.3	
			SoC	8	4 (50.0)	25.00 (31.91)	0.0	16.67	66.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SIHA: Change from baseline in QLQ-C30 Dyspnoea (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Dyspnoea (censored)	>= 4	Baseline	Peg	14	14 (100.0)	14.29 (17.12)	0.0	0.00	33.3	
			SoC	10	10 (100.0)	20.00 (17.21)	0.0	33.33	33.3	
	Week 4	Peg	14	13 (92.9)	7.69 (14.62)	0.0	0.00	33.3		
		SoC	10	10 (100.0)	26.67 (30.63)	0.0	33.33	100.0		
	Week 8	Peg	14	12 (85.7)	8.33 (15.08)	0.0	0.00	33.3		
		SoC	10	10 (100.0)	30.00 (24.60)	0.0	33.33	66.7		
	Week 12	Peg	14	13 (92.9)	5.13 (12.52)	0.0	0.00	33.3		
		SoC	10	10 (100.0)	23.33 (22.50)	0.0	33.33	66.7		
	Week 16	Peg	14	13 (92.9)	7.69 (19.97)	0.0	0.00	66.7		
		SoC	10	10 (100.0)	26.67 (21.08)	0.0	33.33	66.7		
	Week 20	Peg	14	14 (100.0)	7.14 (19.30)	0.0	0.00	66.7		
		SoC	10	10 (100.0)	26.67 (21.08)	0.0	33.33	66.7		
	Week 26	Peg	14	12 (85.7)	8.33 (20.72)	0.0	0.00	66.7		
		SoC	10	9 (90.0)	22.22 (16.67)	0.0	33.33	33.3		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SIHA: Change from baseline in QLQ-C30 Dyspnoea (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in QLQ-C30 Dyspnoea (censored)	Week 4	Peg	21	18 (85.7)	-11.11 (22.87)	-66.7	0.00	33.3	-0.47 (0.54) [-1.58, 0.54]
		SoC	8	5 (62.5)	0.00 (23.57)	-33.3	0.00	33.3	
	Week 8	Peg	21	17 (81.0)	-1.96 (29.98)	-66.7	0.00	66.7	-0.07 (0.54) [-1.13, 0.99]
		SoC	8	5 (62.5)	0.00 (23.57)	-33.3	0.00	33.3	
	Week 12	Peg	21	18 (85.7)	-5.56 (23.57)	-66.7	0.00	33.3	-0.23 (0.53) [-1.31, 0.80]
		SoC	8	5 (62.5)	0.00 (23.57)	-33.3	0.00	33.3	
	Week 16	Peg	21	17 (81.0)	-5.88 (26.97)	-66.7	0.00	33.3	0.20 (0.50) [-0.77, 1.21]
		SoC	8	6 (75.0)	-11.11 (17.21)	-33.3	0.00	0.0	
	Week 20	Peg	21	18 (85.7)	-5.56 (26.20)	-66.7	0.00	33.3	0.04 (0.53) [-1.00, 1.10]
		SoC	8	5 (62.5)	-6.67 (14.91)	-33.3	0.00	0.0	
	Week 26	Peg	21	15 (71.4)	-11.11 (24.12)	-66.7	0.00	33.3	-0.42 (0.68) [-1.83, 0.87]
		SoC	8	3 (37.5)	0.00 (33.33)	-33.3	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QDWC_SIHA: Change from baseline in QLQ-C30 Dyspnoea (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in QLQ-C30 Dyspnoea (censored)	Week 4	Peg	14	13 (92.9)	-7.69 (19.97)	-33.3	0.00	33.3	-0.60 (0.45) [-1.57, 0.23]
		SoC	10	10 (100.0)	6.67 (26.29)	-33.3	0.00	66.7	
	Week 8	Peg	14	12 (85.7)	-5.56 (19.25)	-33.3	0.00	33.3	-0.84 (0.47) [-1.87, 0.00]
		SoC	10	10 (100.0)	10.00 (16.10)	0.0	0.00	33.3	
	Week 12	Peg	14	13 (92.9)	-7.69 (19.97)	-33.3	0.00	33.3	-0.54 (0.45) [-1.50, 0.29]
		SoC	10	10 (100.0)	3.33 (18.92)	-33.3	0.00	33.3	
	Week 16	Peg	14	13 (92.9)	-5.13 (22.96)	-33.3	0.00	33.3	-0.51 (0.45) [-1.46, 0.32]
		SoC	10	10 (100.0)	6.67 (21.08)	-33.3	0.00	33.3	
	Week 20	Peg	14	14 (100.0)	-7.14 (19.30)	-33.3	0.00	33.3	-0.77 (0.45) [-1.74, 0.04]
		SoC	10	10 (100.0)	6.67 (14.05)	0.0	0.00	33.3	
	Week 26	Peg	14	12 (85.7)	-5.56 (23.92)	-33.3	0.00	33.3	-0.45 (0.47) [-1.45, 0.43]
		SoC	10	9 (90.0)	3.70 (11.11)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SICC: Change from baseline in QLQ-C30 Insomnia (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.8231

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SICC: Change from baseline in QLQ-C30 Insomnia (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 60	Peg	Week 4	29	26 (89.7)	-8.23 (4.48)	(-17.34, 0.88)	-6.33 (8.31)	(-23.24, 10.59)	0.4521	
		Week 8	29	24 (82.8)	-7.59 (4.58)	(-16.92, 1.74)	-5.71 (8.27)	(-22.57, 11.16)	0.4952	
		Week 12	29	26 (89.7)	-6.46 (4.64)	(-15.90, 2.99)	-5.95 (8.62)	(-23.50, 11.59)	0.4950	
		Week 16	29	25 (86.2)	-5.20 (4.28)	(-13.91, 3.50)	-4.48 (7.57)	(-19.89, 10.93)	0.5583	
		Week 20	29	27 (93.1)	-2.46 (5.26)	(-13.14, 8.22)	-2.43 (9.89)	(-22.53, 17.67)	0.8072	
		Week 26	29	23 (79.3)	-5.60 (4.89)	(-15.60, 4.40)	4.90 (9.11)	(-13.73, 23.53)	0.5947	
	SoC	Week 4	13	11 (84.6)	-1.90 (6.91)	(-15.95, 12.15)				
		Week 8	13	11 (84.6)	-1.88 (6.78)	(-15.71, 11.94)				
		Week 12	13	11 (84.6)	-0.50 (7.16)	(-15.08, 14.07)				
		Week 16	13	12 (92.3)	-0.72 (6.18)	(-13.30, 11.85)				
		Week 20	13	11 (84.6)	-0.03 (8.29)	(-16.88, 16.83)				
		Week 26	13	10 (76.9)	-10.50 (7.56)	(-25.96, 4.97)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SICC: Change from baseline in QLQ-C30 Insomnia (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	22.10 (10.15)	(-3.99, 48.19)	16.67 (17.63)	(-28.65, 61.99)	0.3879	
		Week 8	6	5 (83.3)	3.94 (8.83)	(-18.76, 26.64)	-8.33 (15.34)	(-47.77, 31.10)	0.6103	
		Week 12	6	5 (83.3)	-7.34 (9.00)	(-30.48, 15.79)	-25.00 (15.63)	(-65.18, 15.18)	0.1707	
		Week 16	6	5 (83.3)	-4.42 (6.26)	(-20.51, 11.67)	-8.33 (10.87)	(-36.29, 19.62)	0.4781	
		Week 20	6	5 (83.3)	-0.26 (8.49)	(-22.08, 21.55)	-8.33 (14.74)	(-46.23, 29.56)	0.5963	
	Week 26	6	4 (66.7)	8.33 (8.56)	(-28.50, 45.17)	16.67 (20.41)	(-71.16, 104.49)	0.5000		
	SoC	Week 4	5	4 (80.0)	5.43 (12.21)	(-25.95, 36.81)				
		Week 8	5	4 (80.0)	12.27 (10.62)	(-15.04, 39.58)				
		Week 12	5	4 (80.0)	17.66 (10.83)	(-10.17, 45.48)				
		Week 16	5	4 (80.0)	3.92 (7.53)	(-15.44, 23.27)				
		Week 20	5	4 (80.0)	8.07 (10.21)	(-18.17, 34.31)				
Week 26	5	2 (40.0)	-8.33 (17.46)	(-83.45, 66.78)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SIHC: Change from baseline in QLQ-C30 Insomnia (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Insomnia (censored)	< 60	Baseline	Peg	29	29 (100.0)	24.14 (26.57)	0.0	33.33	66.7	
			SoC	13	12 (92.3)	30.56 (26.43)	0.0	33.33	66.7	
		Week 4	Peg	29	26 (89.7)	17.95 (25.35)	0.0	0.00	66.7	
			SoC	13	12 (92.3)	22.22 (21.71)	0.0	33.33	66.7	
		Week 8	Peg	29	24 (82.8)	19.44 (23.91)	0.0	0.00	66.7	
			SoC	13	11 (84.6)	24.24 (26.21)	0.0	33.33	66.7	
		Week 12	Peg	29	26 (89.7)	20.51 (26.79)	0.0	0.00	66.7	
			SoC	13	11 (84.6)	24.24 (26.21)	0.0	33.33	66.7	
		Week 16	Peg	29	25 (86.2)	22.67 (26.74)	0.0	0.00	66.7	
			SoC	13	12 (92.3)	27.78 (27.83)	0.0	33.33	66.7	
		Week 20	Peg	29	27 (93.1)	23.46 (33.10)	0.0	0.00	100.0	
			SoC	13	12 (92.3)	25.00 (25.13)	0.0	33.33	66.7	
		Week 26	Peg	29	23 (79.3)	21.74 (27.72)	0.0	0.00	100.0	
			SoC	13	10 (76.9)	16.67 (17.57)	0.0	16.67	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SIHC: Change from baseline in QLQ-C30 Insomnia (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Insomnia (censored)	>= 60	Baseline	Peg	6	6 (100.0)	11.11 (27.22)	0.0	0.00	66.7	
			SoC	5	4 (80.0)	41.67 (16.67)	33.3	33.33	66.7	
		Week 4	Peg	6	5 (83.3)	33.33 (40.82)	0.0	33.33	100.0	
			SoC	5	5 (100.0)	40.00 (27.89)	0.0	33.33	66.7	
		Week 8	Peg	6	5 (83.3)	20.00 (29.81)	0.0	0.00	66.7	
			SoC	5	5 (100.0)	40.00 (27.89)	0.0	33.33	66.7	
		Week 12	Peg	6	5 (83.3)	13.33 (18.26)	0.0	0.00	33.3	
			SoC	5	5 (100.0)	40.00 (27.89)	0.0	33.33	66.7	
		Week 16	Peg	6	5 (83.3)	13.33 (18.26)	0.0	0.00	33.3	
			SoC	5	5 (100.0)	33.33 (23.57)	0.0	33.33	66.7	
		Week 20	Peg	6	5 (83.3)	20.00 (18.26)	0.0	33.33	33.3	
			SoC	5	5 (100.0)	33.33 (23.57)	0.0	33.33	66.7	
		Week 26	Peg	6	4 (66.7)	25.00 (31.91)	0.0	16.67	66.7	
			SoC	5	3 (60.0)	22.22 (19.25)	0.0	33.33	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SIHC: Change from baseline in QLQ-C30 Insomnia (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in QLQ-C30 Insomnia (censored)	Week 4	Peg	29	26 (89.7)	-7.69 (28.76)	-66.7	0.00	66.7	-0.17 (0.37) [-0.91, 0.55]
		SoC	13	11 (84.6)	-3.03 (23.35)	-33.3	0.00	33.3	
	Week 8	Peg	29	24 (82.8)	-6.94 (27.77)	-66.7	0.00	66.7	-0.14 (0.38) [-0.90, 0.59]
		SoC	13	11 (84.6)	-3.03 (23.35)	-33.3	0.00	33.3	
	Week 12	Peg	29	26 (89.7)	-5.13 (29.35)	-66.7	0.00	66.7	-0.07 (0.37) [-0.81, 0.65]
		SoC	13	11 (84.6)	-3.03 (23.35)	-33.3	0.00	33.3	
	Week 16	Peg	29	25 (86.2)	-4.00 (24.19)	-33.3	0.00	66.7	-0.05 (0.36) [-0.77, 0.66]
		SoC	13	12 (92.3)	-2.78 (22.29)	-33.3	0.00	33.3	
	Week 20	Peg	29	27 (93.1)	-2.47 (29.13)	-33.3	0.00	100.0	-0.09 (0.37) [-0.82, 0.63]
		SoC	13	11 (84.6)	0.00 (25.82)	-33.3	0.00	33.3	
	Week 26	Peg	29	23 (79.3)	-5.80 (29.56)	-66.7	0.00	66.7	0.15 (0.39) [-0.61, 0.93]
		SoC	13	10 (76.9)	-10.00 (22.50)	-33.3	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SIHC: Change from baseline in QLQ-C30 Insomnia (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in QLQ-C30 Insomnia (censored)	Week 4	Peg	6	5 (83.3)	20.00 (18.26)	0.0	33.33	33.3	0.59 (0.82) [-0.82, 2.45]
		SoC	5	4 (80.0)	8.33 (16.67)	0.0	0.00	33.3	
	Week 8	Peg	6	5 (83.3)	6.67 (14.91)	0.0	0.00	33.3	-0.09 (0.79) [-1.72, 1.46]
		SoC	5	4 (80.0)	8.33 (16.67)	0.0	0.00	33.3	
	Week 12	Peg	6	5 (83.3)	0.00 (23.57)	-33.3	0.00	33.3	-0.35 (0.80) [-2.10, 1.11]
		SoC	5	4 (80.0)	8.33 (16.67)	0.0	0.00	33.3	
	Week 16	Peg	6	5 (83.3)	0.00 (23.57)	-33.3	0.00	33.3	NE (NE) [NE, NE]
		SoC	5	4 (80.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 20	Peg	6	5 (83.3)	6.67 (27.89)	-33.3	0.00	33.3	0.28 (0.80) [-1.21, 1.99]
		SoC	5	4 (80.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 26	Peg	6	4 (66.7)	8.33 (16.67)	0.0	0.00	33.3	0.46 (1.26) [-1.62, 3.30]
		SoC	5	2 (40.0)	0.00 (0.00)	0.0	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SICD: Change from baseline in QLQ-C30 Insomnia (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA				
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA	
Sex	Peg	Overall									0.8986

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SICD: Change from baseline in QLQ-C30 Insomnia (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Male	Peg	Week 4	19	16 (84.2)	-5.84 (6.14)	(-18.64, 6.96)	-18.22 (10.78)	(-40.70, 4.26)	0.1064	
		Week 8	19	15 (78.9)	-3.50 (6.10)	(-16.27, 9.27)	-16.95 (10.50)	(-38.93, 5.03)	0.1231	
		Week 12	19	17 (89.5)	1.63 (5.71)	(-10.25, 13.51)	-10.04 (10.30)	(-31.46, 11.37)	0.3404	
		Week 16	19	16 (84.2)	-0.33 (5.70)	(-12.18, 11.52)	-7.57 (9.79)	(-27.94, 12.79)	0.4478	
		Week 20	19	17 (89.5)	6.46 (7.08)	(-8.27, 21.19)	-1.81 (12.77)	(-28.36, 24.74)	0.8885	
	Week 26	19	14 (73.7)	3.38 (6.81)	(-11.06, 17.82)	5.71 (12.56)	(-20.92, 32.34)	0.6553		
	SoC	Week 4	10	8 (80.0)	12.38 (8.74)	(-5.85, 30.62)				
		Week 8	10	8 (80.0)	13.45 (8.44)	(-4.20, 31.11)				
		Week 12	10	8 (80.0)	11.68 (8.44)	(-5.88, 29.24)				
		Week 16	10	9 (90.0)	7.25 (7.76)	(-8.90, 23.39)				
		Week 20	10	8 (80.0)	8.27 (10.47)	(-13.50, 30.04)				
Week 26	10	6 (60.0)	-2.33 (10.47)	(-24.52, 19.85)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SICD: Change from baseline in QLQ-C30 Insomnia (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Female	Peg	Week 4	16	15 (93.8)	-4.83 (5.89)	(-17.20, 7.53)	-2.08 (12.48)	(-28.31, 24.14)	0.8693	
		Week 8	16	14 (87.5)	-7.93 (4.93)	(-18.32, 2.46)	7.19 (10.41)	(-14.77, 29.14)	0.4990	
		Week 12	16	14 (87.5)	-15.19 (5.84)	(-27.52, -2.87)	-8.85 (12.34)	(-34.88, 17.19)	0.4831	
		Week 16	16	14 (87.5)	-9.63 (4.74)	(-19.63, 0.38)	-0.47 (10.02)	(-21.61, 20.66)	0.9628	
		Week 20	16	15 (93.8)	-10.56 (5.19)	(-21.45, 0.34)	-3.12 (11.00)	(-26.23, 19.98)	0.7795	
	Week 26	16	13 (81.3)	-11.46 (5.28)	(-22.73, -0.20)	3.16 (11.23)	(-20.78, 27.10)	0.7820		
	SoC	Week 4	8	7 (87.5)	-2.75 (10.34)	(-24.47, 18.97)				
		Week 8	8	7 (87.5)	-15.11 (8.34)	(-32.72, 2.49)				
		Week 12	8	7 (87.5)	-6.35 (9.89)	(-27.22, 14.53)				
		Week 16	8	7 (87.5)	-9.16 (8.03)	(-26.10, 7.79)				
		Week 20	8	7 (87.5)	-7.43 (9.11)	(-26.57, 11.71)				
Week 26	8	6 (75.0)	-14.63 (9.24)	(-34.33, 5.07)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SIHD: Change from baseline in QLQ-C30 Insomnia (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Insomnia (censored)	Male	Baseline	Peg	19	19 (100.0)	17.54 (25.74)	0.0	0.00	66.7	
			SoC	10	9 (90.0)	37.04 (26.06)	0.0	33.33	66.7	
		Week 4	Peg	19	16 (84.2)	16.67 (24.34)	0.0	0.00	66.7	
			SoC	10	9 (90.0)	33.33 (23.57)	0.0	33.33	66.7	
		Week 8	Peg	19	15 (78.9)	20.00 (24.56)	0.0	0.00	66.7	
			SoC	10	9 (90.0)	37.04 (26.06)	0.0	33.33	66.7	
		Week 12	Peg	19	17 (89.5)	23.53 (28.30)	0.0	0.00	66.7	
			SoC	10	9 (90.0)	37.04 (26.06)	0.0	33.33	66.7	
		Week 16	Peg	19	16 (84.2)	22.92 (29.11)	0.0	0.00	66.7	
			SoC	10	10 (100.0)	36.67 (24.60)	0.0	33.33	66.7	
		Week 20	Peg	19	17 (89.5)	27.45 (35.81)	0.0	0.00	100.0	
			SoC	10	9 (90.0)	33.33 (23.57)	0.0	33.33	66.7	
		Week 26	Peg	19	14 (73.7)	28.57 (31.64)	0.0	33.33	100.0	
			SoC	10	7 (70.0)	23.81 (16.27)	0.0	33.33	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SIHD: Change from baseline in QLQ-C30 Insomnia (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Insomnia (censored)	Female	Baseline	Peg	16	16 (100.0)	27.08 (27.81)	0.0	33.33	66.7	
			SoC	8	7 (87.5)	28.57 (23.00)	0.0	33.33	66.7	
		Week 4	Peg	16	15 (93.8)	24.44 (32.04)	0.0	0.00	100.0	
			SoC	8	8 (100.0)	20.83 (24.80)	0.0	16.67	66.7	
		Week 8	Peg	16	14 (87.5)	19.05 (25.20)	0.0	0.00	66.7	
			SoC	8	7 (87.5)	19.05 (26.23)	0.0	0.00	66.7	
		Week 12	Peg	16	14 (87.5)	14.29 (21.54)	0.0	0.00	66.7	
			SoC	8	7 (87.5)	19.05 (26.23)	0.0	0.00	66.7	
		Week 16	Peg	16	14 (87.5)	19.05 (21.54)	0.0	16.67	66.7	
			SoC	8	7 (87.5)	19.05 (26.23)	0.0	0.00	66.7	
		Week 20	Peg	16	15 (93.8)	17.78 (24.77)	0.0	0.00	66.7	
			SoC	8	8 (100.0)	20.83 (24.80)	0.0	16.67	66.7	
		Week 26	Peg	16	13 (81.3)	15.38 (22.01)	0.0	0.00	66.7	
			SoC	8	6 (75.0)	11.11 (17.21)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SIHD: Change from baseline in QLQ-C30 Insomnia (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Insomnia (censored)	Male	Week 4	Peg	19	16 (84.2)	-2.08 (33.26)	-66.7	0.00	66.7	-0.19 (0.46) [-1.11, 0.69]
			SoC	10	8 (80.0)	4.17 (27.82)	-33.3	0.00	33.3	
		Week 8	Peg	19	15 (78.9)	0.00 (30.86)	-66.7	0.00	66.7	-0.28 (0.46) [-1.23, 0.60]
			SoC	10	8 (80.0)	8.33 (23.57)	-33.3	0.00	33.3	
		Week 12	Peg	19	17 (89.5)	3.92 (26.04)	-33.3	0.00	66.7	-0.17 (0.45) [-1.07, 0.70]
			SoC	10	8 (80.0)	8.33 (23.57)	-33.3	0.00	33.3	
		Week 16	Peg	19	16 (84.2)	2.08 (25.73)	-33.3	0.00	66.7	-0.07 (0.44) [-0.93, 0.79]
			SoC	10	9 (90.0)	3.70 (20.03)	-33.3	0.00	33.3	
		Week 20	Peg	19	17 (89.5)	7.84 (32.34)	-33.3	0.00	100.0	0.12 (0.45) [-0.75, 1.02]
			SoC	10	8 (80.0)	4.17 (21.36)	-33.3	0.00	33.3	
		Week 26	Peg	19	14 (73.7)	4.76 (28.81)	-33.3	0.00	66.7	0.36 (0.52) [-0.63, 1.43]
			SoC	10	6 (60.0)	-5.56 (25.09)	-33.3	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SIHD: Change from baseline in QLQ-C30 Insomnia (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Insomnia (censored)	Female	Week 4	Peg	16	15 (93.8)	-4.44 (24.77)	-66.7	0.00	33.3	0.01 (0.48) [-0.94, 0.97]
			SoC	8	7 (87.5)	-4.76 (12.60)	-33.3	0.00	0.0	
		Week 8	Peg	16	14 (87.5)	-9.52 (20.37)	-66.7	0.00	0.0	NE (NE) [NE, NE]
			SoC	8	7 (87.5)	-9.52 (16.27)	-33.3	0.00	0.0	
		Week 12	Peg	16	14 (87.5)	-14.29 (28.39)	-66.7	0.00	33.3	-0.18 (0.49) [-1.17, 0.77]
			SoC	8	7 (87.5)	-9.52 (16.27)	-33.3	0.00	0.0	
		Week 16	Peg	16	14 (87.5)	-9.52 (20.37)	-33.3	0.00	33.3	NE (NE) [NE, NE]
			SoC	8	7 (87.5)	-9.52 (16.27)	-33.3	0.00	0.0	
		Week 20	Peg	16	15 (93.8)	-11.11 (20.57)	-33.3	0.00	33.3	-0.29 (0.49) [-1.28, 0.64]
			SoC	8	7 (87.5)	-4.76 (23.00)	-33.3	0.00	33.3	
		Week 26	Peg	16	13 (81.3)	-12.82 (25.60)	-66.7	0.00	33.3	-0.07 (0.53) [-1.12, 0.96]
			SoC	8	6 (75.0)	-11.11 (17.21)	-33.3	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SICB: Change from baseline in QLQ-C30 Insomnia (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.7048

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SICB: Change from baseline in QLQ-C30 Insomnia (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Latin America	Peg	Week 4	12	11 (91.7)	-10.83 (9.25)	(-31.76, 10.11)	-18.52 (24.64)	(-74.27, 37.23)	0.4716	
		Week 8	12	10 (83.3)	-10.79 (8.04)	(-29.33, 7.75)	-14.74 (20.88)	(-62.89, 33.40)	0.5001	
		Week 12	12	11 (91.7)	-12.71 (6.42)	(-27.22, 1.81)	-15.20 (17.09)	(-53.86, 23.45)	0.3967	
		Week 16	12	10 (83.3)	-8.13 (4.41)	(-18.30, 2.04)	-15.44 (11.45)	(-41.85, 10.97)	0.2146	
		Week 20	12	10 (83.3)	0.99 (6.96)	(-15.06, 17.05)	-10.71 (18.08)	(-52.41, 30.99)	0.5699	
		Week 26	12	9 (75.0)	-1.47 (10.76)	(-26.90, 23.96)	-7.91 (27.07)	(-71.92, 56.11)	0.7787	
	SoC	Week 4	2	2 (100.0)	7.69 (22.48)	(-43.16, 58.54)				
		Week 8	2	2 (100.0)	3.95 (18.85)	(-39.51, 47.42)				
		Week 12	2	2 (100.0)	2.50 (15.59)	(-32.76, 37.75)				
		Week 16	2	2 (100.0)	7.31 (10.34)	(-16.53, 31.15)				
		Week 20	2	2 (100.0)	11.70 (16.32)	(-25.94, 49.35)				
		Week 26	2	2 (100.0)	6.44 (24.09)	(-50.52, 63.40)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SICB: Change from baseline in QLQ-C30 Insomnia (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Asia Pacific Peg	Peg	Week 4	23	20 (87.0)	-1.34 (5.25)	(-12.09, 9.40)	-3.38 (9.05)	(-21.89, 15.13)	0.7116	
		Week 8	23	19 (82.6)	-2.88 (5.30)	(-13.75, 7.98)	-4.53 (9.04)	(-23.06, 14.00)	0.6203	
		Week 12	23	20 (87.0)	-2.41 (5.47)	(-13.61, 8.78)	-6.59 (9.43)	(-25.87, 12.69)	0.4902	
		Week 16	23	20 (87.0)	-3.37 (5.29)	(-14.17, 7.44)	-3.41 (8.86)	(-21.51, 14.69)	0.7028	
		Week 20	23	22 (95.7)	-4.72 (5.87)	(-16.70, 7.26)	-7.69 (10.38)	(-28.86, 13.47)	0.4641	
	Week 26	23	18 (78.3)	-5.89 (4.87)	(-15.95, 4.16)	4.07 (9.20)	(-14.91, 23.06)	0.6619		
	SoC	Week 4	16	13 (81.3)	2.04 (6.77)	(-11.82, 15.89)				
		Week 8	16	13 (81.3)	1.65 (6.59)	(-11.86, 15.15)				
		Week 12	16	13 (81.3)	4.17 (7.06)	(-10.26, 18.61)				
		Week 16	16	14 (87.5)	0.05 (6.47)	(-13.16, 13.26)				
Week 20		16	13 (81.3)	2.97 (7.95)	(-13.24, 19.19)					
Week 26	16	10 (62.5)	-9.97 (7.32)	(-25.07, 5.14)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SIHB: Change from baseline in QLQ-C30 Insomnia (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Insomnia (censored)	Latin America	Baseline	Peg	12	12 (100.0)	25.00 (32.18)	0.0	0.00	66.7	
			SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
		Week 4	Peg	12	11 (91.7)	15.15 (34.52)	0.0	0.00	100.0	
	SoC		2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3		
		Week 8	Peg	12	10 (83.3)	16.67 (28.33)	0.0	0.00	66.7	
	SoC		2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3		
		Week 12	Peg	12	11 (91.7)	12.12 (22.47)	0.0	0.00	66.7	
	SoC		2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3		
		Week 16	Peg	12	10 (83.3)	20.00 (23.31)	0.0	16.67	66.7	
	SoC		2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3		
		Week 20	Peg	12	10 (83.3)	30.00 (33.15)	0.0	33.33	100.0	
	SoC		2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3		
		Week 26	Peg	12	9 (75.0)	29.63 (35.14)	0.0	33.33	100.0	
	SoC		2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SIHB: Change from baseline in QLQ-C30 Insomnia (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Insomnia (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	20.29 (24.08)	0.0	0.00	66.7	
			SoC	16	14 (87.5)	38.10 (22.10)	0.0	33.33	66.7	
		Week 4	Peg	23	20 (87.0)	23.33 (24.42)	0.0	33.33	66.7	
			SoC	16	15 (93.8)	28.89 (24.77)	0.0	33.33	66.7	
		Week 8	Peg	23	19 (82.6)	21.05 (22.80)	0.0	33.33	66.7	
			SoC	16	14 (87.5)	30.95 (27.62)	0.0	33.33	66.7	
		Week 12	Peg	23	20 (87.0)	23.33 (26.71)	0.0	16.67	66.7	
			SoC	16	14 (87.5)	30.95 (27.62)	0.0	33.33	66.7	
		Week 16	Peg	23	20 (87.0)	21.67 (27.09)	0.0	0.00	66.7	
			SoC	16	15 (93.8)	31.11 (26.63)	0.0	33.33	66.7	
		Week 20	Peg	23	22 (95.7)	19.70 (30.27)	0.0	0.00	100.0	
			SoC	16	15 (93.8)	28.89 (24.77)	0.0	33.33	66.7	
		Week 26	Peg	23	18 (78.3)	18.52 (23.49)	0.0	0.00	66.7	
			SoC	16	11 (68.8)	18.18 (17.41)	0.0	33.33	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SIHB: Change from baseline in QLQ-C30 Insomnia (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Insomnia (censored)	Latin America	Week 4	Peg	12	11 (91.7)	-12.12 (30.81)	-66.7	0.00	33.3	-0.89 (0.88) [-2.83, 0.66]
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 8	Peg	12	10 (83.3)	-13.33 (28.11)	-66.7	0.00	0.0	-1.00 (0.91) [-3.05, 0.55]
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 12	Peg	12	11 (91.7)	-15.15 (27.34)	-66.7	0.00	0.0	-1.10 (0.90) [-3.12, 0.43]
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 16	Peg	12	10 (83.3)	-10.00 (16.10)	-33.3	0.00	0.0	-1.45 (0.95) [-3.69, 0.07]
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 20	Peg	12	10 (83.3)	0.00 (22.22)	-33.3	0.00	33.3	-0.69 (0.89) [-2.62, 0.90]
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 26	Peg	12	9 (75.0)	-3.70 (30.93)	-66.7	0.00	33.3	-0.62 (0.91) [-2.59, 1.01]
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SIHB: Change from baseline in QLQ-C30 Insomnia (censored) by region
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Insomnia (censored)	Week 4	Peg	23	20 (87.0)	1.67 (27.52)	-33.3	0.00	66.7	0.16 (0.37) [-0.55, 0.90]
		SoC	16	13 (81.3)	-2.56 (21.35)	-33.3	0.00	33.3	
	Week 8	Peg	23	19 (82.6)	0.00 (24.85)	-33.3	0.00	66.7	0.11 (0.37) [-0.62, 0.85]
		SoC	16	13 (81.3)	-2.56 (21.35)	-33.3	0.00	33.3	
	Week 12	Peg	23	20 (87.0)	1.67 (27.52)	-33.3	0.00	66.7	0.16 (0.37) [-0.55, 0.90]
		SoC	16	13 (81.3)	-2.56 (21.35)	-33.3	0.00	33.3	
	Week 16	Peg	23	20 (87.0)	0.00 (26.49)	-33.3	0.00	66.7	0.20 (0.36) [-0.50, 0.92]
		SoC	16	14 (87.5)	-4.76 (17.82)	-33.3	0.00	33.3	
	Week 20	Peg	23	22 (95.7)	-1.52 (31.67)	-33.3	0.00	100.0	0.04 (0.36) [-0.67, 0.75]
		SoC	16	13 (81.3)	-2.56 (21.35)	-33.3	0.00	33.3	
	Week 26	Peg	23	18 (78.3)	-3.70 (27.75)	-33.3	0.00	66.7	0.38 (0.41) [-0.40, 1.23]
		SoC	16	10 (62.5)	-13.33 (17.21)	-33.3	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SICA: Change from baseline in QLQ-C30 Insomnia (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.4375

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SICA: Change from baseline in QLQ-C30 Insomnia (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 4	21	18 (85.7)	-6.86 (5.80)	(-18.96, 5.24)	-11.55 (12.61)	(-37.85, 14.75)	0.3706	
		Week 8	21	17 (81.0)	-9.11 (4.49)	(-18.50, 0.28)	-20.09 (9.51)	(-39.99, -0.18)	0.0481	*
		Week 12	21	18 (85.7)	-7.10 (5.15)	(-17.84, 3.64)	-26.00 (11.19)	(-49.34, -2.66)	0.0308	*
		Week 16	21	17 (81.0)	-7.62 (4.59)	(-17.19, 1.95)	-18.10 (9.18)	(-37.24, 1.05)	0.0626	
		Week 20	21	18 (85.7)	-8.60 (4.61)	(-18.21, 1.01)	-19.56 (10.01)	(-40.45, 1.33)	0.0649	
	Week 26	21	15 (71.4)	-10.35 (5.33)	(-21.72, 1.02)	-6.54 (13.22)	(-34.72, 21.65)	0.6282		
	SoC	Week 4	8	5 (62.5)	4.69 (11.11)	(-18.49, 27.87)				
		Week 8	8	5 (62.5)	10.98 (8.33)	(-6.47, 28.42)				
		Week 12	8	5 (62.5)	18.90 (9.86)	(-1.67, 39.47)				
		Week 16	8	6 (75.0)	10.48 (7.83)	(-5.86, 26.81)				
		Week 20	8	5 (62.5)	10.96 (8.83)	(-7.45, 29.37)				
Week 26	8	3 (37.5)	-3.81 (12.04)	(-29.48, 21.85)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SICA: Change from baseline in QLQ-C30 Insomnia (censored) by number of transfusions previous year
ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	-2.10 (6.81)	(-16.30, 12.10)	-4.83 (10.43)	(-26.58, 16.92)	0.6482	
		Week 8	14	12 (85.7)	-2.23 (7.18)	(-17.25, 12.79)	-1.57 (10.77)	(-24.12, 20.98)	0.8855	
		Week 12	14	13 (92.9)	-4.62 (6.37)	(-17.90, 8.67)	-0.62 (9.76)	(-20.97, 19.74)	0.9503	
		Week 16	14	13 (92.9)	-1.54 (5.82)	(-13.69, 10.61)	3.13 (8.92)	(-15.48, 21.73)	0.7297	
		Week 20	14	14 (100.0)	6.02 (7.79)	(-10.18, 22.21)	7.77 (12.16)	(-17.52, 33.06)	0.5297	
		Week 26	14	12 (85.7)	2.34 (6.92)	(-12.19, 16.88)	9.17 (10.58)	(-13.05, 31.40)	0.3973	
	SoC	Week 4	10	10 (100.0)	2.73 (7.78)	(-13.50, 18.96)				
		Week 8	10	10 (100.0)	-0.66 (7.88)	(-17.14, 15.83)				
		Week 12	10	10 (100.0)	-4.00 (7.28)	(-19.19, 11.19)				
		Week 16	10	10 (100.0)	-4.66 (6.65)	(-18.55, 9.22)				
		Week 20	10	10 (100.0)	-1.76 (9.24)	(-20.96, 17.45)				
		Week 26	10	9 (90.0)	-6.83 (7.99)	(-23.62, 9.96)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
ANCOVA: fixed effects = treatment, covariate = baseline.
Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SIHA: Change from baseline in QLQ-C30 Insomnia (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Insomnia (censored)	< 4	Baseline	Peg	21	21 (100.0)	23.81 (28.17)	0.0	0.00	66.7	
			SoC	8	6 (75.0)	44.44 (27.22)	0.0	50.00	66.7	
		Week 4	Peg	21	18 (85.7)	22.22 (30.25)	0.0	0.00	100.0	
			SoC	8	7 (87.5)	28.57 (23.00)	0.0	33.33	66.7	
		Week 8	Peg	21	17 (81.0)	21.57 (23.40)	0.0	33.33	66.7	
			SoC	8	6 (75.0)	38.89 (25.09)	0.0	33.33	66.7	
		Week 12	Peg	21	18 (85.7)	22.22 (25.57)	0.0	16.67	66.7	
			SoC	8	6 (75.0)	44.44 (27.22)	0.0	50.00	66.7	
		Week 16	Peg	21	17 (81.0)	23.53 (25.72)	0.0	33.33	66.7	
			SoC	8	7 (87.5)	42.86 (25.20)	0.0	33.33	66.7	
		Week 20	Peg	21	18 (85.7)	20.37 (25.92)	0.0	0.00	66.7	
			SoC	8	7 (87.5)	33.33 (27.22)	0.0	33.33	66.7	
		Week 26	Peg	21	15 (71.4)	22.22 (24.12)	0.0	33.33	66.7	
			SoC	8	4 (50.0)	25.00 (16.67)	0.0	33.33	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SIHA: Change from baseline in QLQ-C30 Insomnia (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Insomnia (censored)	>= 4	Baseline	Peg	14	14 (100.0)	19.05 (25.20)	0.0	0.00	66.7	
			SoC	10	10 (100.0)	26.67 (21.08)	0.0	33.33	66.7	
	Week 4		Peg	14	13 (92.9)	17.95 (25.88)	0.0	0.00	66.7	
			SoC	10	10 (100.0)	26.67 (26.29)	0.0	33.33	66.7	
	Week 8		Peg	14	12 (85.7)	16.67 (26.59)	0.0	0.00	66.7	
			SoC	10	10 (100.0)	23.33 (27.44)	0.0	16.67	66.7	
	Week 12		Peg	14	13 (92.9)	15.38 (25.88)	0.0	0.00	66.7	
			SoC	10	10 (100.0)	20.00 (23.31)	0.0	16.67	66.7	
	Week 16		Peg	14	13 (92.9)	17.95 (25.88)	0.0	0.00	66.7	
			SoC	10	10 (100.0)	20.00 (23.31)	0.0	16.67	66.7	
	Week 20		Peg	14	14 (100.0)	26.19 (37.39)	0.0	0.00	100.0	
			SoC	10	10 (100.0)	23.33 (22.50)	0.0	33.33	66.7	
	Week 26		Peg	14	12 (85.7)	22.22 (32.82)	0.0	0.00	100.0	
			SoC	10	9 (90.0)	14.81 (17.57)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SIHA: Change from baseline in QLQ-C30 Insomnia (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in QLQ-C30 Insomnia (censored)	Week 4	Peg	21	18 (85.7)	-5.56 (26.20)	-66.7	0.00	33.3	-0.19 (0.53) [-1.27, 0.84]
		SoC	8	5 (62.5)	0.00 (33.33)	-33.3	0.00	33.3	
	Week 8	Peg	21	17 (81.0)	-7.84 (22.14)	-66.7	0.00	33.3	-0.60 (0.55) [-1.75, 0.41]
		SoC	8	5 (62.5)	6.67 (27.89)	-33.3	0.00	33.3	
	Week 12	Peg	21	18 (85.7)	-5.56 (26.20)	-66.7	0.00	33.3	-0.68 (0.54) [-1.83, 0.32]
		SoC	8	5 (62.5)	13.33 (29.81)	-33.3	33.33	33.3	
	Week 16	Peg	21	17 (81.0)	-5.88 (21.20)	-33.3	0.00	33.3	-0.50 (0.51) [-1.55, 0.45]
		SoC	8	6 (75.0)	5.56 (25.09)	-33.3	0.00	33.3	
	Week 20	Peg	21	18 (85.7)	-7.41 (21.56)	-33.3	0.00	33.3	-0.59 (0.54) [-1.73, 0.41]
		SoC	8	5 (62.5)	6.67 (27.89)	-33.3	0.00	33.3	
	Week 26	Peg	21	15 (71.4)	-8.89 (26.63)	-66.7	0.00	33.3	0.07 (0.68) [-1.26, 1.43]
		SoC	8	3 (37.5)	-11.11 (38.49)	-33.3	-33.33	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QIWC_SIHA: Change from baseline in QLQ-C30 Insomnia (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in QLQ-C30 Insomnia (censored)	Week 4	Peg	14	13 (92.9)	0.00 (33.33)	-66.7	0.00	66.7	NE (NE) [NE, NE]
		SoC	10	10 (100.0)	0.00 (15.71)	-33.3	0.00	33.3	
	Week 8	Peg	14	12 (85.7)	0.00 (31.78)	-66.7	0.00	66.7	0.12 (0.45) [-0.76, 1.03]
		SoC	10	10 (100.0)	-3.33 (18.92)	-33.3	0.00	33.3	
	Week 12	Peg	14	13 (92.9)	-2.56 (31.80)	-66.7	0.00	66.7	0.15 (0.44) [-0.71, 1.05]
		SoC	10	10 (100.0)	-6.67 (14.05)	-33.3	0.00	0.0	
	Week 16	Peg	14	13 (92.9)	0.00 (27.22)	-33.3	0.00	66.7	0.29 (0.44) [-0.56, 1.20]
		SoC	10	10 (100.0)	-6.67 (14.05)	-33.3	0.00	0.0	
	Week 20	Peg	14	14 (100.0)	7.14 (35.03)	-33.3	0.00	100.0	0.34 (0.44) [-0.49, 1.24]
		SoC	10	10 (100.0)	-3.33 (18.92)	-33.3	0.00	33.3	
	Week 26	Peg	14	12 (85.7)	2.78 (30.01)	-33.3	0.00	66.7	0.40 (0.47) [-0.49, 1.38]
		SoC	10	9 (90.0)	-7.41 (14.70)	-33.3	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SICC: Change from baseline in QLQ-C30 Appetite Loss (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.7086

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SICC: Change from baseline in QLQ-C30 Appetite Loss (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 60	Peg	Week 4	29	26 (89.7)	-7.19 (3.20)	(-13.71, -0.68)	-12.11 (6.00)	(-24.31, 0.09)	0.0516	
		Week 8	29	24 (82.8)	-1.80 (3.85)	(-9.65, 6.04)	-12.11 (7.08)	(-26.56, 2.33)	0.0973	
		Week 12	29	26 (89.7)	-0.52 (4.52)	(-9.71, 8.67)	-4.99 (8.53)	(-22.34, 12.36)	0.5622	
		Week 16	29	25 (86.2)	-0.57 (4.78)	(-10.30, 9.17)	-10.30 (8.53)	(-27.66, 7.06)	0.2361	
		Week 20	29	27 (93.1)	-3.36 (4.08)	(-11.65, 4.94)	-11.59 (7.76)	(-27.37, 4.19)	0.1447	
		Week 26	29	23 (79.3)	-1.46 (3.66)	(-8.94, 6.03)	-7.66 (6.91)	(-21.80, 6.48)	0.2768	
	SoC	Week 4	13	11 (84.6)	4.91 (4.96)	(-5.18, 15.00)				
		Week 8	13	11 (84.6)	10.31 (5.75)	(-1.42, 22.03)				
		Week 12	13	11 (84.6)	4.47 (7.03)	(-9.82, 18.77)				
		Week 16	13	12 (92.3)	9.73 (6.94)	(-4.38, 23.84)				
		Week 20	13	11 (84.6)	8.24 (6.48)	(-4.94, 21.41)				
		Week 26	13	10 (76.9)	6.21 (5.71)	(-5.48, 17.89)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SICC: Change from baseline in QLQ-C30 Appetite Loss (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	-5.37 (16.27)	(-47.20, 36.46)	-36.90 (25.87)	(-103.39, 29.60)	0.2131	
		Week 8	6	5 (83.3)	-4.42 (10.89)	(-32.42, 23.57)	-16.79 (17.32)	(-61.30, 27.72)	0.3767	
		Week 12	6	5 (83.3)	-12.16 (12.69)	(-44.79, 20.47)	-36.13 (20.18)	(-88.01, 15.74)	0.1334	
		Week 16	6	5 (83.3)	-13.42 (10.33)	(-39.96, 13.13)	-39.95 (16.41)	(-82.14, 2.25)	0.0591	
		Week 20	6	5 (83.3)	-13.42 (10.33)	(-39.96, 13.13)	-39.95 (16.41)	(-82.14, 2.25)	0.0591	
	Week 26	6	4 (66.7)	-22.50 (7.93)	(-56.61, 11.61)	-15.00 (16.20)	(-84.71, 54.71)	0.4523		
	SoC	Week 4	5	4 (80.0)	31.52 (19.20)	(-17.83, 80.88)				
		Week 8	5	4 (80.0)	12.37 (12.85)	(-20.67, 45.41)				
		Week 12	5	4 (80.0)	23.98 (14.98)	(-14.53, 62.48)				
		Week 16	5	4 (80.0)	26.53 (12.18)	(-4.79, 57.85)				
		Week 20	5	4 (80.0)	26.53 (12.18)	(-4.79, 57.85)				
Week 26	5	2 (40.0)	-7.50 (14.30)	(-69.03, 54.03)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SIHC: Change from baseline in QLQ-C30 Appetite Loss (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Appetite Loss (censored)	< 60	Baseline	Peg	29	29 (100.0)	10.34 (15.69)	0.0	0.00	33.3	
			SoC	13	12 (92.3)	13.89 (17.16)	0.0	0.00	33.3	
		Week 4	Peg	29	26 (89.7)	5.13 (12.26)	0.0	0.00	33.3	
			SoC	13	12 (92.3)	16.67 (22.47)	0.0	0.00	66.7	
		Week 8	Peg	29	24 (82.8)	8.33 (14.74)	0.0	0.00	33.3	
			SoC	13	11 (84.6)	24.24 (26.21)	0.0	33.33	66.7	
		Week 12	Peg	29	26 (89.7)	10.26 (20.59)	0.0	0.00	66.7	
			SoC	13	11 (84.6)	18.18 (27.34)	0.0	0.00	66.7	
		Week 16	Peg	29	25 (86.2)	9.33 (20.46)	0.0	0.00	66.7	
			SoC	13	12 (92.3)	22.22 (29.59)	0.0	0.00	66.7	
		Week 20	Peg	29	27 (93.1)	8.64 (17.52)	0.0	0.00	66.7	
			SoC	13	12 (92.3)	19.44 (26.43)	0.0	0.00	66.7	
		Week 26	Peg	29	23 (79.3)	8.70 (14.97)	0.0	0.00	33.3	
			SoC	13	10 (76.9)	20.00 (23.31)	0.0	16.67	66.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SIHC: Change from baseline in QLQ-C30 Appetite Loss (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Appetite Loss (censored)	>= 60	Baseline	Peg	6	6 (100.0)	16.67 (40.82)	0.0	0.00	100.0	
			SoC	5	4 (80.0)	16.67 (19.25)	0.0	16.67	33.3	
		Week 4	Peg	6	5 (83.3)	13.33 (18.26)	0.0	0.00	33.3	
			SoC	5	5 (100.0)	46.67 (38.01)	0.0	33.33	100.0	
		Week 8	Peg	6	5 (83.3)	13.33 (18.26)	0.0	0.00	33.3	
			SoC	5	5 (100.0)	26.67 (27.89)	0.0	33.33	66.7	
		Week 12	Peg	6	5 (83.3)	6.67 (14.91)	0.0	0.00	33.3	
			SoC	5	5 (100.0)	33.33 (33.33)	0.0	33.33	66.7	
		Week 16	Peg	6	5 (83.3)	6.67 (14.91)	0.0	0.00	33.3	
			SoC	5	5 (100.0)	33.33 (33.33)	0.0	33.33	66.7	
		Week 20	Peg	6	5 (83.3)	6.67 (14.91)	0.0	0.00	33.3	
			SoC	5	5 (100.0)	33.33 (33.33)	0.0	33.33	66.7	
		Week 26	Peg	6	4 (66.7)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	5	3 (60.0)	11.11 (19.25)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SIHC: Change from baseline in QLQ-C30 Appetite Loss (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in QLQ-C30 Appetite Loss (censored)	Week 4	Peg	29	26 (89.7)	-6.41 (16.38)	-33.3	0.00	33.3	-0.46 (0.37) [-1.22, 0.25]
		SoC	13	11 (84.6)	3.03 (27.71)	-33.3	0.00	66.7	
	Week 8	Peg	29	24 (82.8)	-1.39 (15.48)	-33.3	0.00	33.3	-0.49 (0.38) [-1.27, 0.23]
		SoC	13	11 (84.6)	9.09 (30.15)	-33.3	0.00	66.7	
	Week 12	Peg	29	26 (89.7)	0.00 (21.08)	-33.3	0.00	66.7	-0.12 (0.37) [-0.86, 0.60]
		SoC	13	11 (84.6)	3.03 (31.46)	-33.3	0.00	66.7	
	Week 16	Peg	29	25 (86.2)	0.00 (19.25)	-33.3	0.00	66.7	-0.32 (0.36) [-1.06, 0.37]
		SoC	13	12 (92.3)	8.33 (35.18)	-33.3	0.00	66.7	
	Week 20	Peg	29	27 (93.1)	-2.47 (24.33)	-33.3	0.00	66.7	-0.31 (0.37) [-1.06, 0.40]
		SoC	13	11 (84.6)	6.06 (32.72)	-33.3	0.00	66.7	
	Week 26	Peg	29	23 (79.3)	-1.45 (15.82)	-33.3	0.00	33.3	-0.37 (0.39) [-1.18, 0.38]
		SoC	13	10 (76.9)	6.67 (30.63)	-33.3	0.00	66.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SIHC: Change from baseline in QLQ-C30 Appetite Loss (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in QLQ-C30 Appetite Loss (censored)	Week 4	Peg	6	5 (83.3)	-6.67 (36.51)	-66.7	0.00	33.3	-0.86 (0.85) [-2.88, 0.50]
		SoC	5	4 (80.0)	33.33 (47.14)	0.0	16.67	100.0	
	Week 8	Peg	6	5 (83.3)	-6.67 (36.51)	-66.7	0.00	33.3	-0.59 (0.82) [-2.45, 0.82]
		SoC	5	4 (80.0)	16.67 (33.33)	0.0	0.00	66.7	
	Week 12	Peg	6	5 (83.3)	-13.33 (50.55)	-100.0	0.00	33.3	-0.78 (0.84) [-2.75, 0.59]
		SoC	5	4 (80.0)	25.00 (31.91)	0.0	16.67	66.7	
	Week 16	Peg	6	5 (83.3)	-13.33 (29.81)	-66.7	0.00	0.0	-1.11 (0.89) [-3.29, 0.22]
		SoC	5	4 (80.0)	25.00 (31.91)	0.0	16.67	66.7	
	Week 20	Peg	6	5 (83.3)	-13.33 (29.81)	-66.7	0.00	0.0	-1.11 (0.89) [-3.29, 0.22]
		SoC	5	4 (80.0)	25.00 (31.91)	0.0	16.67	66.7	
	Week 26	Peg	6	4 (66.7)	-25.00 (50.00)	-100.0	0.00	0.0	-0.46 (1.26) [-3.30, 1.62]
		SoC	5	2 (40.0)	0.00 (0.00)	0.0	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SICD: Change from baseline in QLQ-C30 Appetite Loss (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.2722

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SICD: Change from baseline in QLQ-C30 Appetite Loss (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Male	Peg	Week 4	19	16 (84.2)	-4.35 (4.47)	(-13.68, 4.98)	-14.53 (8.00)	(-31.23, 2.16)	0.0844	
		Week 8	19	15 (78.9)	0.41 (4.24)	(-8.47, 9.29)	-13.77 (7.60)	(-29.69, 2.15)	0.0860	
		Week 12	19	17 (89.5)	0.51 (5.57)	(-11.08, 12.10)	-9.18 (10.30)	(-30.59, 12.24)	0.3831	
		Week 16	19	16 (84.2)	-0.28 (6.66)	(-14.14, 13.58)	-17.32 (11.67)	(-41.58, 6.94)	0.1525	
		Week 20	19	17 (89.5)	-0.49 (5.83)	(-12.63, 11.64)	-11.90 (10.78)	(-34.32, 10.52)	0.2821	
		Week 26	19	14 (73.7)	1.54 (3.93)	(-6.80, 9.88)	-0.43 (7.30)	(-15.91, 15.06)	0.9542	
	SoC	Week 4	10	8 (80.0)	10.18 (6.38)	(-3.13, 23.50)				
		Week 8	10	8 (80.0)	14.18 (5.95)	(1.73, 26.63)				
		Week 12	10	8 (80.0)	9.68 (8.29)	(-7.56, 26.93)				
		Week 16	10	9 (90.0)	17.04 (9.14)	(-1.97, 36.05)				
		Week 20	10	8 (80.0)	11.41 (8.68)	(-6.65, 29.46)				
		Week 26	10	6 (60.0)	1.97 (6.07)	(-10.90, 14.83)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SICD: Change from baseline in QLQ-C30 Appetite Loss (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Female	Peg	Week 4	16	15 (93.8)	-9.71 (6.70)	(-23.79, 4.36)	-27.78 (14.18)	(-57.56, 2.00)	0.0657	
		Week 8	16	14 (87.5)	-5.07 (6.25)	(-18.26, 8.12)	-14.77 (13.28)	(-42.80, 13.26)	0.2817	
		Week 12	16	14 (87.5)	-6.96 (7.00)	(-21.73, 7.81)	-24.38 (14.87)	(-55.76, 7.00)	0.1196	
		Week 16	16	14 (87.5)	-6.52 (5.96)	(-19.10, 6.06)	-21.20 (12.67)	(-47.93, 5.53)	0.1125	
		Week 20	16	15 (93.8)	-9.65 (5.39)	(-20.97, 1.66)	-28.19 (11.40)	(-52.13, -4.25)	0.0236	*
		Week 26	16	13 (81.3)	-11.97 (5.04)	(-22.71, -1.22)	-22.33 (10.87)	(-45.50, 0.85)	0.0579	
	SoC	Week 4	8	7 (87.5)	18.07 (11.77)	(-6.65, 42.79)				
		Week 8	8	7 (87.5)	9.70 (10.68)	(-12.82, 32.23)				
		Week 12	8	7 (87.5)	17.42 (11.96)	(-7.80, 42.65)				
		Week 16	8	7 (87.5)	14.69 (10.18)	(-6.80, 36.17)				
		Week 20	8	7 (87.5)	18.53 (9.46)	(-1.34, 38.40)				
		Week 26	8	6 (75.0)	10.36 (8.95)	(-8.72, 29.45)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SIHD: Change from baseline in QLQ-C30 Appetite Loss (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Appetite Loss (censored)	Male	Baseline	Peg	19	19 (100.0)	7.02 (13.96)	0.0	0.00	33.3	
			SoC	10	9 (90.0)	18.52 (17.57)	0.0	33.33	33.3	
		Week 4	Peg	19	16 (84.2)	6.25 (13.44)	0.0	0.00	33.3	
			SoC	10	9 (90.0)	25.93 (22.22)	0.0	33.33	66.7	
		Week 8	Peg	19	15 (78.9)	8.89 (15.26)	0.0	0.00	33.3	
			SoC	10	9 (90.0)	25.93 (22.22)	0.0	33.33	66.7	
		Week 12	Peg	19	17 (89.5)	9.80 (19.60)	0.0	0.00	66.7	
			SoC	10	9 (90.0)	22.22 (28.87)	0.0	0.00	66.7	
		Week 16	Peg	19	16 (84.2)	8.33 (22.77)	0.0	0.00	66.7	
			SoC	10	10 (100.0)	26.67 (30.63)	0.0	16.67	66.7	
		Week 20	Peg	19	17 (89.5)	9.80 (19.60)	0.0	0.00	66.7	
			SoC	10	9 (90.0)	22.22 (28.87)	0.0	0.00	66.7	
		Week 26	Peg	19	14 (73.7)	11.90 (16.57)	0.0	0.00	33.3	
			SoC	10	7 (70.0)	14.29 (17.82)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SIHD: Change from baseline in QLQ-C30 Appetite Loss (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Appetite Loss (censored)	Female	Baseline	Peg	16	16 (100.0)	16.67 (27.22)	0.0	0.00	100.0	
			SoC	8	7 (87.5)	9.52 (16.27)	0.0	0.00	33.3	
		Week 4	Peg	16	15 (93.8)	6.67 (13.80)	0.0	0.00	33.3	
			SoC	8	8 (100.0)	25.00 (38.83)	0.0	0.00	100.0	
		Week 8	Peg	16	14 (87.5)	9.52 (15.63)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	23.81 (31.71)	0.0	0.00	66.7	
		Week 12	Peg	16	14 (87.5)	9.52 (20.37)	0.0	0.00	66.7	
			SoC	8	7 (87.5)	23.81 (31.71)	0.0	0.00	66.7	
		Week 16	Peg	16	14 (87.5)	9.52 (15.63)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	23.81 (31.71)	0.0	0.00	66.7	
		Week 20	Peg	16	15 (93.8)	6.67 (13.80)	0.0	0.00	33.3	
			SoC	8	8 (100.0)	25.00 (29.55)	0.0	16.67	66.7	
		Week 26	Peg	16	13 (81.3)	2.56 (9.25)	0.0	0.00	33.3	
			SoC	8	6 (75.0)	22.22 (27.22)	0.0	16.67	66.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SIHD: Change from baseline in QLQ-C30 Appetite Loss (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Appetite Loss (censored)	Male	Week 4	Peg	19	16 (84.2)	-2.08 (19.12)	-33.3	0.00	33.3	-0.30 (0.46) [-1.24, 0.57]
			SoC	10	8 (80.0)	4.17 (21.36)	-33.3	0.00	33.3	
		Week 8	Peg	19	15 (78.9)	2.22 (15.26)	-33.3	0.00	33.3	-0.32 (0.46) [-1.27, 0.56]
			SoC	10	8 (80.0)	8.33 (23.57)	-33.3	0.00	33.3	
		Week 12	Peg	19	17 (89.5)	1.96 (21.96)	-33.3	0.00	66.7	-0.09 (0.45) [-0.99, 0.79]
			SoC	10	8 (80.0)	4.17 (27.82)	-33.3	0.00	33.3	
		Week 16	Peg	19	16 (84.2)	2.08 (22.67)	-33.3	0.00	66.7	-0.32 (0.44) [-1.23, 0.51]
			SoC	10	9 (90.0)	11.11 (33.33)	-33.3	0.00	66.7	
		Week 20	Peg	19	17 (89.5)	1.96 (24.92)	-33.3	0.00	66.7	-0.08 (0.45) [-0.98, 0.80]
			SoC	10	8 (80.0)	4.17 (27.82)	-33.3	0.00	33.3	
		Week 26	Peg	19	14 (73.7)	2.38 (15.82)	-33.3	0.00	33.3	0.13 (0.52) [-0.88, 1.17]
			SoC	10	6 (60.0)	0.00 (21.08)	-33.3	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SIHD: Change from baseline in QLQ-C30 Appetite Loss (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Appetite Loss (censored)	Female	Week 4	Peg	16	15 (93.8)	-11.11 (20.57)	-66.7	0.00	0.0	-0.94 (0.51) [-2.06, -0.05]
			SoC	8	7 (87.5)	19.05 (46.58)	-33.3	0.00	100.0	
		Week 8	Peg	16	14 (87.5)	-7.14 (23.31)	-66.7	0.00	33.3	-0.72 (0.51) [-1.81, 0.19]
			SoC	8	7 (87.5)	14.29 (37.80)	-33.3	0.00	66.7	
		Week 12	Peg	16	14 (87.5)	-7.14 (32.50)	-100.0	0.00	33.3	-0.60 (0.50) [-1.67, 0.32]
			SoC	8	7 (87.5)	14.29 (37.80)	-33.3	0.00	66.7	
		Week 16	Peg	16	14 (87.5)	-7.14 (19.30)	-66.7	0.00	0.0	-0.77 (0.51) [-1.87, 0.14]
			SoC	8	7 (87.5)	14.29 (37.80)	-33.3	0.00	66.7	
		Week 20	Peg	16	15 (93.8)	-11.11 (24.12)	-66.7	0.00	33.3	-1.00 (0.51) [-2.13, -0.11]
			SoC	8	7 (87.5)	19.05 (37.80)	-33.3	0.00	66.7	
		Week 26	Peg	16	13 (81.3)	-12.82 (28.99)	-100.0	0.00	0.0	-0.74 (0.54) [-1.93, 0.23]
			SoC	8	6 (75.0)	11.11 (34.43)	-33.3	0.00	66.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SICB: Change from baseline in QLQ-C30 Appetite Loss (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.7892

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SICB: Change from baseline in QLQ-C30 Appetite Loss (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Latin America	Peg	Week 4	12	11 (91.7)	-17.31 (3.15)	(-24.43, -10.18)	-14.53 (8.02)	(-32.67, 3.61)	0.1033	
		Week 8	12	10 (83.3)	-9.65 (4.29)	(-19.55, 0.24)	-7.91 (10.52)	(-32.17, 16.36)	0.4740	
		Week 12	12	11 (91.7)	-16.93 (3.35)	(-24.51, -9.36)	-13.50 (8.52)	(-32.77, 5.78)	0.1476	
		Week 16	12	10 (83.3)	-9.73 (5.82)	(-23.15, 3.68)	-8.39 (14.26)	(-41.28, 24.50)	0.5724	
		Week 20	12	10 (83.3)	-12.70 (4.15)	(-22.27, -3.13)	-9.52 (10.22)	(-33.09, 14.04)	0.3786	
		Week 26	12	9 (75.0)	-12.33 (5.95)	(-26.40, 1.73)	-5.15 (13.99)	(-38.23, 27.94)	0.7239	
	SoC	Week 4	2	2 (100.0)	-2.77 (7.38)	(-19.46, 13.91)				
		Week 8	2	2 (100.0)	-1.75 (9.60)	(-23.89, 20.40)				
		Week 12	2	2 (100.0)	-3.43 (7.84)	(-21.17, 14.30)				
		Week 16	2	2 (100.0)	-1.34 (13.02)	(-31.36, 28.68)				
		Week 20	2	2 (100.0)	-3.17 (9.32)	(-24.66, 18.31)				
		Week 26	2	2 (100.0)	-7.19 (12.67)	(-37.14, 22.77)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SICB: Change from baseline in QLQ-C30 Appetite Loss (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Asia Pacific Peg	Peg	Week 4	23	20 (87.0)	-1.59 (5.42)	(-12.68, 9.51)	-16.81 (9.16)	(-35.54, 1.92)	0.0767	
		Week 8	23	19 (82.6)	0.57 (4.85)	(-9.36, 10.49)	-13.99 (8.20)	(-30.78, 2.80)	0.0989	
		Week 12	23	20 (87.0)	5.63 (5.95)	(-6.55, 17.80)	-6.13 (10.26)	(-27.11, 14.86)	0.5550	
		Week 16	23	20 (87.0)	-0.53 (5.68)	(-12.14, 11.07)	-17.96 (9.30)	(-36.96, 1.03)	0.0630	
		Week 20	23	22 (95.7)	-2.80 (5.18)	(-13.36, 7.76)	-20.43 (9.03)	(-38.84, -2.02)	0.0308	*
	Week 26	23	18 (78.3)	-1.33 (4.00)	(-9.59, 6.93)	-9.77 (7.42)	(-25.07, 5.54)	0.2003		
	SoC	Week 4	16	13 (81.3)	15.22 (6.93)	(1.04, 29.40)				
		Week 8	16	13 (81.3)	14.56 (6.01)	(2.25, 26.86)				
		Week 12	16	13 (81.3)	11.76 (7.68)	(-3.95, 27.46)				
		Week 16	16	14 (87.5)	17.43 (6.90)	(3.35, 31.51)				
Week 20		16	13 (81.3)	17.63 (6.96)	(3.43, 31.84)					
Week 26	16	10 (62.5)	8.44 (5.90)	(-3.74, 20.61)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SIHb: Change from baseline in QLQ-C30 Appetite Loss (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Appetite Loss (censored)	Latin America	Baseline	Peg	12	12 (100.0)	19.44 (30.01)	0.0	0.00	100.0	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 4	Peg	12	11 (91.7)	3.03 (10.05)	0.0	0.00	33.3	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 8	Peg	12	10 (83.3)	10.00 (16.10)	0.0	0.00	33.3	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 12	Peg	12	11 (91.7)	3.03 (10.05)	0.0	0.00	33.3	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 16	Peg	12	10 (83.3)	10.00 (22.50)	0.0	0.00	66.7	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 20	Peg	12	10 (83.3)	10.00 (16.10)	0.0	0.00	33.3	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 26	Peg	12	9 (75.0)	11.11 (16.67)	0.0	0.00	33.3	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SIHB: Change from baseline in QLQ-C30 Appetite Loss (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Appetite Loss (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	7.25 (14.06)	0.0	0.00	33.3	
			SoC	16	14 (87.5)	14.29 (17.12)	0.0	0.00	33.3	
		Week 4	Peg	23	20 (87.0)	8.33 (14.81)	0.0	0.00	33.3	
			SoC	16	15 (93.8)	26.67 (31.37)	0.0	33.33	100.0	
		Week 8	Peg	23	19 (82.6)	8.77 (15.08)	0.0	0.00	33.3	
			SoC	16	14 (87.5)	26.19 (26.73)	0.0	33.33	66.7	
		Week 12	Peg	23	20 (87.0)	13.33 (22.69)	0.0	0.00	66.7	
			SoC	16	14 (87.5)	23.81 (30.46)	0.0	0.00	66.7	
		Week 16	Peg	23	20 (87.0)	8.33 (18.34)	0.0	0.00	66.7	
			SoC	16	15 (93.8)	26.67 (31.37)	0.0	0.00	66.7	
		Week 20	Peg	23	22 (95.7)	7.58 (17.61)	0.0	0.00	66.7	
			SoC	16	15 (93.8)	24.44 (29.46)	0.0	0.00	66.7	
		Week 26	Peg	23	18 (78.3)	5.56 (12.78)	0.0	0.00	33.3	
			SoC	16	11 (68.8)	18.18 (22.92)	0.0	0.00	66.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SIHb: Change from baseline in QLQ-C30 Appetite Loss (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Appetite Loss (censored)	Latin America	Week 4	Peg	12	11 (91.7)	-18.18 (22.92)	-66.7	0.00	0.0	-0.65 (0.87) [-2.52, 0.92]
			SoC	2	2 (100.0)	0.00 (47.14)	-33.3	0.00	33.3	
		Week 8	Peg	12	10 (83.3)	-10.00 (27.44)	-66.7	0.00	33.3	-0.31 (0.87) [-2.12, 1.35]
			SoC	2	2 (100.0)	0.00 (47.14)	-33.3	0.00	33.3	
		Week 12	Peg	12	11 (91.7)	-18.18 (31.14)	-100.0	0.00	0.0	-0.51 (0.86) [-2.34, 1.08]
			SoC	2	2 (100.0)	0.00 (47.14)	-33.3	0.00	33.3	
		Week 16	Peg	12	10 (83.3)	-10.00 (27.44)	-66.7	0.00	33.3	-0.31 (0.87) [-2.12, 1.35]
			SoC	2	2 (100.0)	0.00 (47.14)	-33.3	0.00	33.3	
		Week 20	Peg	12	10 (83.3)	-13.33 (28.11)	-66.7	0.00	33.3	-0.40 (0.87) [-2.24, 1.23]
			SoC	2	2 (100.0)	0.00 (47.14)	-33.3	0.00	33.3	
		Week 26	Peg	12	9 (75.0)	-14.81 (37.68)	-100.0	0.00	33.3	-0.35 (0.89) [-2.23, 1.33]
			SoC	2	2 (100.0)	0.00 (47.14)	-33.3	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SIHb: Change from baseline in QLQ-C30 Appetite Loss (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Appetite Loss (censored)	Asia	Week 4	Peg	23	20 (87.0)	0.00 (15.29)	-33.3	0.00	33.3	-0.51 (0.37) [-1.28, 0.20]
			SoC	16	13 (81.3)	12.82 (34.80)	-33.3	0.00	100.0	
	Pacific	Week 8	Peg	23	19 (82.6)	1.75 (13.49)	-33.3	0.00	33.3	-0.51 (0.38) [-1.30, 0.20]
			SoC	16	13 (81.3)	12.82 (28.99)	-33.3	0.00	66.7	
		Week 12	Peg	23	20 (87.0)	6.67 (20.52)	-33.3	0.00	66.7	-0.14 (0.37) [-0.88, 0.58]
			SoC	16	13 (81.3)	10.26 (31.58)	-33.3	0.00	66.7	
		Week 16	Peg	23	20 (87.0)	1.67 (17.01)	-33.3	0.00	66.7	-0.49 (0.37) [-1.24, 0.20]
			SoC	16	14 (87.5)	14.29 (33.88)	-33.3	0.00	66.7	
		Week 20	Peg	23	22 (95.7)	0.00 (23.00)	-33.3	0.00	66.7	-0.47 (0.37) [-1.22, 0.22]
			SoC	16	13 (81.3)	12.82 (32.03)	-33.3	0.00	66.7	
		Week 26	Peg	23	18 (78.3)	0.00 (11.43)	-33.3	0.00	33.3	-0.36 (0.41) [-1.21, 0.43]
			SoC	16	10 (62.5)	6.67 (26.29)	-33.3	0.00	66.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SICA: Change from baseline in QLQ-C30 Appetite Loss (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.5293

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SICA: Change from baseline in QLQ-C30 Appetite Loss (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 4	21	18 (85.7)	-12.16 (3.54)	(-19.55, -4.77)	-15.94 (7.66)	(-31.92, 0.03)	0.0504	
		Week 8	21	17 (81.0)	-10.98 (3.66)	(-18.65, -3.32)	-15.00 (7.75)	(-31.22, 1.23)	0.0681	
		Week 12	21	18 (85.7)	-10.71 (5.58)	(-22.35, 0.93)	-15.94 (12.07)	(-41.11, 9.23)	0.2014	
		Week 16	21	17 (81.0)	-10.60 (5.04)	(-21.11, -0.09)	-23.95 (9.89)	(-44.59, -3.32)	0.0251	*
		Week 20	21	18 (85.7)	-12.48 (4.76)	(-22.41, -2.54)	-17.39 (10.30)	(-38.88, 4.09)	0.1069	
	Week 26	21	15 (71.4)	-13.65 (3.74)	(-21.63, -5.67)	-4.11 (9.17)	(-23.67, 15.44)	0.6604		
	SoC	Week 4	8	5 (62.5)	3.78 (6.76)	(-10.32, 17.88)				
		Week 8	8	5 (62.5)	4.01 (6.80)	(-10.21, 18.24)				
		Week 12	8	5 (62.5)	5.23 (10.65)	(-16.99, 27.45)				
		Week 16	8	6 (75.0)	13.36 (8.50)	(-4.37, 31.08)				
		Week 20	8	5 (62.5)	4.91 (9.09)	(-14.05, 23.88)				
Week 26	8	3 (37.5)	-9.54 (8.37)	(-27.39, 8.31)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SICA: Change from baseline in QLQ-C30 Appetite Loss (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	-2.51 (6.95)	(-17.02, 11.99)	-22.45 (10.64)	(-44.64, -0.26)	0.0477	*
		Week 8	14	12 (85.7)	5.34 (6.46)	(-8.18, 18.86)	-14.92 (9.79)	(-35.41, 5.58)	0.1441	
		Week 12	14	13 (92.9)	5.09 (6.91)	(-9.33, 19.52)	-11.62 (10.75)	(-34.04, 10.80)	0.2925	
		Week 16	14	13 (92.9)	6.16 (7.31)	(-9.09, 21.42)	-9.16 (11.37)	(-32.88, 14.55)	0.4299	
		Week 20	14	14 (100.0)	2.60 (6.34)	(-10.58, 15.77)	-17.10 (9.92)	(-37.74, 3.53)	0.0994	
		Week 26	14	12 (85.7)	2.90 (5.71)	(-9.10, 14.90)	-11.75 (8.98)	(-30.62, 7.13)	0.2075	
	SoC	Week 4	10	10 (100.0)	19.93 (7.94)	(3.36, 36.51)				
		Week 8	10	10 (100.0)	20.26 (7.10)	(5.39, 35.12)				
		Week 12	10	10 (100.0)	16.71 (7.93)	(0.17, 33.25)				
		Week 16	10	10 (100.0)	15.32 (8.39)	(-2.17, 32.82)				
		Week 20	10	10 (100.0)	19.70 (7.52)	(4.06, 35.34)				
		Week 26	10	9 (90.0)	14.65 (6.65)	(0.69, 28.61)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SIHA: Change from baseline in QLQ-C30 Appetite Loss (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Appetite Loss (censored)	< 4	Baseline	Peg	21	21 (100.0)	15.87 (24.99)	0.0	0.00	100.0	
			SoC	8	6 (75.0)	22.22 (17.21)	0.0	33.33	33.3	
		Week 4	Peg	21	18 (85.7)	7.41 (14.26)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	23.81 (25.20)	0.0	33.33	66.7	
		Week 8	Peg	21	17 (81.0)	7.84 (14.57)	0.0	0.00	33.3	
			SoC	8	6 (75.0)	22.22 (27.22)	0.0	16.67	66.7	
		Week 12	Peg	21	18 (85.7)	9.26 (19.15)	0.0	0.00	66.7	
			SoC	8	6 (75.0)	22.22 (34.43)	0.0	0.00	66.7	
		Week 16	Peg	21	17 (81.0)	7.84 (14.57)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	28.57 (35.63)	0.0	0.00	66.7	
		Week 20	Peg	21	18 (85.7)	7.41 (14.26)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	19.05 (32.53)	0.0	0.00	66.7	
		Week 26	Peg	21	15 (71.4)	6.67 (13.80)	0.0	0.00	33.3	
			SoC	8	4 (50.0)	8.33 (16.67)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SIHA: Change from baseline in QLQ-C30 Appetite Loss (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Appetite Loss (censored)	>= 4	Baseline	Peg	14	14 (100.0)	4.76 (12.10)	0.0	0.00	33.3	
			SoC	10	10 (100.0)	10.00 (16.10)	0.0	0.00	33.3	
	Week 4		Peg	14	13 (92.9)	5.13 (12.52)	0.0	0.00	33.3	
			SoC	10	10 (100.0)	26.67 (34.43)	0.0	16.67	100.0	
	Week 8		Peg	14	12 (85.7)	11.11 (16.41)	0.0	0.00	33.3	
			SoC	10	10 (100.0)	26.67 (26.29)	0.0	33.33	66.7	
	Week 12		Peg	14	13 (92.9)	10.26 (21.01)	0.0	0.00	66.7	
			SoC	10	10 (100.0)	23.33 (27.44)	0.0	16.67	66.7	
	Week 16		Peg	14	13 (92.9)	10.26 (25.04)	0.0	0.00	66.7	
			SoC	10	10 (100.0)	23.33 (27.44)	0.0	16.67	66.7	
	Week 20		Peg	14	14 (100.0)	9.52 (20.37)	0.0	0.00	66.7	
			SoC	10	10 (100.0)	26.67 (26.29)	0.0	33.33	66.7	
	Week 26		Peg	14	12 (85.7)	8.33 (15.08)	0.0	0.00	33.3	
			SoC	10	9 (90.0)	22.22 (23.57)	0.0	33.33	66.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SIHA: Change from baseline in QLQ-C30 Appetite Loss (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in QLQ-C30 Appetite Loss (censored)	Week 4	Peg	21	18 (85.7)	-11.11 (19.80)	-66.7	0.00	0.0	-0.52 (0.54) [-1.64, 0.49]
		SoC	8	5 (62.5)	0.00 (23.57)	-33.3	0.00	33.3	
	Week 8	Peg	21	17 (81.0)	-9.80 (19.60)	-66.7	0.00	0.0	-0.46 (0.54) [-1.59, 0.56]
		SoC	8	5 (62.5)	0.00 (23.57)	-33.3	0.00	33.3	
	Week 12	Peg	21	18 (85.7)	-9.26 (29.83)	-100.0	0.00	33.3	-0.29 (0.53) [-1.38, 0.73]
		SoC	8	5 (62.5)	0.00 (33.33)	-33.3	0.00	33.3	
	Week 16	Peg	21	17 (81.0)	-9.80 (19.60)	-66.7	0.00	0.0	-0.77 (0.52) [-1.88, 0.16]
		SoC	8	6 (75.0)	11.11 (40.37)	-33.3	16.67	66.7	
	Week 20	Peg	21	18 (85.7)	-11.11 (25.57)	-66.7	0.00	33.3	-0.39 (0.54) [-1.50, 0.62]
		SoC	8	5 (62.5)	0.00 (33.33)	-33.3	0.00	33.3	
	Week 26	Peg	21	15 (71.4)	-13.33 (27.60)	-100.0	0.00	0.0	-0.08 (0.68) [-1.43, 1.25]
		SoC	8	3 (37.5)	-11.11 (19.25)	-33.3	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QAWC_SIHA: Change from baseline in QLQ-C30 Appetite Loss (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in QLQ-C30 Appetite Loss (censored)	Week 4	Peg	14	13 (92.9)	0.00 (19.25)	-33.3	0.00	33.3	-0.54 (0.45) [-1.50, 0.29]
		SoC	10	10 (100.0)	16.67 (39.28)	-33.3	0.00	100.0	
	Week 8	Peg	14	12 (85.7)	8.33 (15.08)	0.0	0.00	33.3	-0.33 (0.45) [-1.27, 0.53]
		SoC	10	10 (100.0)	16.67 (32.39)	-33.3	0.00	66.7	
	Week 12	Peg	14	13 (92.9)	7.69 (19.97)	0.0	0.00	66.7	-0.21 (0.44) [-1.11, 0.64]
		SoC	10	10 (100.0)	13.33 (32.20)	-33.3	0.00	66.7	
	Week 16	Peg	14	13 (92.9)	7.69 (19.97)	0.0	0.00	66.7	-0.21 (0.44) [-1.11, 0.64]
		SoC	10	10 (100.0)	13.33 (32.20)	-33.3	0.00	66.7	
	Week 20	Peg	14	14 (100.0)	4.76 (22.10)	-33.3	0.00	66.7	-0.43 (0.44) [-1.34, 0.40]
		SoC	10	10 (100.0)	16.67 (32.39)	-33.3	0.00	66.7	
	Week 26	Peg	14	12 (85.7)	5.56 (12.97)	0.0	0.00	33.3	-0.25 (0.47) [-1.21, 0.64]
		SoC	10	9 (90.0)	11.11 (28.87)	-33.3	0.00	66.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SICC: Change from baseline in QLQ-C30 Constipation (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.7668

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SICC: Change from baseline in QLQ-C30 Constipation (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 60	Peg	Week 4	29	26 (89.7)	-2.12 (2.98)	(-8.18, 3.95)	-1.17 (5.55)	(-12.47, 10.12)	0.8343	
		Week 8	29	24 (82.8)	-2.33 (3.22)	(-8.91, 4.24)	-7.56 (5.85)	(-19.50, 4.37)	0.2059	
		Week 12	29	26 (89.7)	0.55 (3.41)	(-6.38, 7.49)	7.99 (6.37)	(-4.97, 20.94)	0.2184	
		Week 16	29	25 (86.2)	3.73 (3.78)	(-3.96, 11.41)	6.02 (6.74)	(-7.70, 19.74)	0.3782	
		Week 20	29	27 (93.1)	1.30 (3.48)	(-5.78, 8.37)	1.46 (6.58)	(-11.92, 14.83)	0.8264	
		Week 26	29	23 (79.3)	-3.76 (3.31)	(-10.52, 3.00)	-2.53 (6.20)	(-15.20, 10.15)	0.6863	
	SoC	Week 4	13	11 (84.6)	-0.95 (4.61)	(-10.33, 8.44)				
		Week 8	13	11 (84.6)	5.23 (4.79)	(-4.54, 15.00)				
		Week 12	13	11 (84.6)	-7.44 (5.28)	(-18.18, 3.31)				
		Week 16	13	12 (92.3)	-2.30 (5.49)	(-13.47, 8.87)				
		Week 20	13	11 (84.6)	-0.16 (5.51)	(-11.35, 11.04)				
		Week 26	13	10 (76.9)	-1.23 (5.13)	(-11.71, 9.25)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SICC: Change from baseline in QLQ-C30 Constipation (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	-2.83 (10.37)	(-29.48, 23.82)	0.98 (17.46)	(-43.90, 45.86)	0.9574	
		Week 8	6	5 (83.3)	6.43 (5.07)	(-6.60, 19.45)	17.65 (8.53)	(-4.28, 39.58)	0.0934	
		Week 12	6	5 (83.3)	0.33 (4.91)	(-12.28, 12.94)	10.78 (8.26)	(-10.45, 32.02)	0.2486	
		Week 16	6	5 (83.3)	1.08 (4.80)	(-11.27, 13.42)	12.37 (7.91)	(-7.97, 32.71)	0.1789	
		Week 20	6	5 (83.3)	8.78 (8.34)	(-12.66, 30.23)	20.43 (13.74)	(-14.90, 55.76)	0.1973	
		Week 26	6	4 (66.7)	6.94 (5.97)	(-18.76, 32.65)	16.67 (11.79)	(-34.04, 67.37)	0.2929	
	SoC	Week 4	5	4 (80.0)	-3.81 (12.09)	(-34.89, 27.26)				
		Week 8	5	4 (80.0)	-11.22 (5.91)	(-26.41, 3.97)				
		Week 12	5	4 (80.0)	-10.46 (5.72)	(-25.16, 4.25)				
		Week 16	5	4 (80.0)	-11.29 (5.64)	(-25.80, 3.22)				
		Week 20	5	4 (80.0)	-11.65 (9.80)	(-36.85, 13.55)				
		Week 26	5	2 (40.0)	-9.72 (10.25)	(-53.84, 34.39)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SIHC: Change from baseline in QLQ-C30 Constipation (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Constipation < 60 (censored)	Baseline	Peg	29	29 (100.0)	12.64 (18.72)	0.0	0.00	66.7	
		SoC	13	12 (92.3)	5.56 (12.97)	0.0	0.00	33.3	
	Week 4	Peg	29	26 (89.7)	7.69 (14.32)	0.0	0.00	33.3	
		SoC	13	12 (92.3)	8.33 (15.08)	0.0	0.00	33.3	
	Week 8	Peg	29	24 (82.8)	8.33 (14.74)	0.0	0.00	33.3	
		SoC	13	11 (84.6)	15.15 (17.41)	0.0	0.00	33.3	
	Week 12	Peg	29	26 (89.7)	11.54 (18.72)	0.0	0.00	66.7	
		SoC	13	11 (84.6)	3.03 (10.05)	0.0	0.00	33.3	
	Week 16	Peg	29	25 (86.2)	14.67 (19.44)	0.0	0.00	66.7	
		SoC	13	12 (92.3)	8.33 (15.08)	0.0	0.00	33.3	
	Week 20	Peg	29	27 (93.1)	12.35 (18.83)	0.0	0.00	66.7	
		SoC	13	12 (92.3)	8.33 (15.08)	0.0	0.00	33.3	
	Week 26	Peg	29	23 (79.3)	8.70 (14.97)	0.0	0.00	33.3	
		SoC	13	10 (76.9)	10.00 (16.10)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SIHC: Change from baseline in QLQ-C30 Constipation (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Constipation >= 60 (censored)	Baseline	Peg	6	6 (100.0)	11.11 (17.21)	0.0	0.00	33.3	
		SoC	5	4 (80.0)	33.33 (27.22)	0.0	33.33	66.7	
	Week 4	Peg	6	5 (83.3)	13.33 (18.26)	0.0	0.00	33.3	
		SoC	5	5 (100.0)	13.33 (18.26)	0.0	0.00	33.3	
	Week 8	Peg	6	5 (83.3)	13.33 (18.26)	0.0	0.00	33.3	
		SoC	5	5 (100.0)	20.00 (29.81)	0.0	0.00	66.7	
	Week 12	Peg	6	5 (83.3)	6.67 (14.91)	0.0	0.00	33.3	
		SoC	5	5 (100.0)	20.00 (29.81)	0.0	0.00	66.7	
	Week 16	Peg	6	5 (83.3)	13.33 (18.26)	0.0	0.00	33.3	
		SoC	5	5 (100.0)	20.00 (29.81)	0.0	0.00	66.7	
	Week 20	Peg	6	5 (83.3)	20.00 (29.81)	0.0	0.00	66.7	
		SoC	5	5 (100.0)	20.00 (29.81)	0.0	0.00	66.7	
	Week 26	Peg	6	4 (66.7)	16.67 (19.25)	0.0	16.67	33.3	
		SoC	5	3 (60.0)	11.11 (19.25)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SIHC: Change from baseline in QLQ-C30 Constipation (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in QLQ-C30 Constipation (censored)	Week 4	Peg	29	26 (89.7)	-3.85 (23.71)	-33.3	0.00	33.3	-0.30 (0.37) [-1.06, 0.41]
		SoC	13	11 (84.6)	3.03 (17.98)	-33.3	0.00	33.3	
	Week 8	Peg	29	24 (82.8)	-4.17 (20.41)	-33.3	0.00	33.3	-0.62 (0.38) [-1.42, 0.09]
		SoC	13	11 (84.6)	9.09 (21.56)	-33.3	0.00	33.3	
	Week 12	Peg	29	26 (89.7)	-1.28 (25.79)	-66.7	0.00	33.3	0.07 (0.37) [-0.65, 0.81]
		SoC	13	11 (84.6)	-3.03 (17.98)	-33.3	0.00	33.3	
	Week 16	Peg	29	25 (86.2)	1.33 (26.32)	-33.3	0.00	66.7	-0.06 (0.36) [-0.77, 0.65]
		SoC	13	12 (92.3)	2.78 (22.29)	-33.3	0.00	33.3	
	Week 20	Peg	29	27 (93.1)	0.00 (24.46)	-33.3	0.00	66.7	-0.12 (0.37) [-0.86, 0.60]
		SoC	13	11 (84.6)	3.03 (23.35)	-33.3	0.00	33.3	
	Week 26	Peg	29	23 (79.3)	-5.80 (23.89)	-66.7	0.00	33.3	-0.39 (0.40) [-1.20, 0.36]
		SoC	13	10 (76.9)	3.33 (18.92)	-33.3	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SIHC: Change from baseline in QLQ-C30 Constipation (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in QLQ-C30 Constipation (censored)	Week 4	Peg	6	5 (83.3)	6.67 (14.91)	0.0	0.00	33.3	0.84 (0.85) [-0.51, 2.85]
		SoC	5	4 (80.0)	-16.67 (33.33)	-66.7	0.00	0.0	
	Week 8	Peg	6	5 (83.3)	6.67 (14.91)	0.0	0.00	33.3	0.85 (0.85) [-0.51, 2.86]
		SoC	5	4 (80.0)	-8.33 (16.67)	-33.3	0.00	0.0	
	Week 12	Peg	6	5 (83.3)	0.00 (0.00)	0.0	0.00	0.0	0.68 (0.83) [-0.71, 2.59]
		SoC	5	4 (80.0)	-8.33 (16.67)	-33.3	0.00	0.0	
	Week 16	Peg	6	5 (83.3)	0.00 (0.00)	0.0	0.00	0.0	0.68 (0.83) [-0.71, 2.59]
		SoC	5	4 (80.0)	-8.33 (16.67)	-33.3	0.00	0.0	
	Week 20	Peg	6	5 (83.3)	6.67 (14.91)	0.0	0.00	33.3	0.85 (0.85) [-0.51, 2.86]
		SoC	5	4 (80.0)	-8.33 (16.67)	-33.3	0.00	0.0	
	Week 26	Peg	6	4 (66.7)	8.33 (16.67)	0.0	0.00	33.3	0.46 (1.26) [-1.62, 3.30]
		SoC	5	2 (40.0)	0.00 (0.00)	0.0	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SICD: Change from baseline in QLQ-C30 Constipation (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.0861

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SICD: Change from baseline in QLQ-C30 Constipation (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Male	Peg	Week 4	19	16 (84.2)	-1.58 (3.71)	(-9.32, 6.16)	-12.36 (6.53)	(-25.98, 1.27)	0.0731	
		Week 8	19	15 (78.9)	0.64 (3.68)	(-7.06, 8.34)	-5.93 (6.35)	(-19.23, 7.37)	0.3622	
		Week 12	19	17 (89.5)	-1.70 (3.73)	(-9.45, 6.05)	1.09 (6.70)	(-12.85, 15.03)	0.8725	
		Week 16	19	16 (84.2)	3.43 (5.07)	(-7.10, 13.97)	4.87 (8.56)	(-12.93, 22.68)	0.5753	
		Week 20	19	17 (89.5)	0.57 (5.62)	(-11.12, 12.27)	-2.61 (10.02)	(-23.46, 18.23)	0.7969	
		Week 26	19	14 (73.7)	-1.36 (4.52)	(-10.94, 8.21)	-10.10 (8.30)	(-27.69, 7.49)	0.2410	
	SoC	Week 4	10	8 (80.0)	10.78 (5.32)	(-0.32, 21.87)				
		Week 8	10	8 (80.0)	6.57 (5.12)	(-4.14, 17.28)				
		Week 12	10	8 (80.0)	-2.79 (5.53)	(-14.28, 8.70)				
		Week 16	10	9 (90.0)	-1.44 (6.89)	(-15.77, 12.90)				
		Week 20	10	8 (80.0)	3.19 (8.29)	(-14.05, 20.42)				
		Week 26	10	6 (60.0)	8.74 (6.92)	(-5.94, 23.41)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SICD: Change from baseline in QLQ-C30 Constipation (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Female	Peg	Week 4	16	15 (93.8)	-3.82 (3.48)	(-11.13, 3.49)	5.50 (7.34)	(-9.93, 20.93)	0.4639	
		Week 8	16	14 (87.5)	-7.85 (4.16)	(-16.63, 0.94)	-20.99 (8.75)	(-39.46, -2.53)	0.0282	*
		Week 12	16	14 (87.5)	-2.62 (5.72)	(-14.68, 9.44)	-4.69 (12.01)	(-30.03, 20.65)	0.7012	
		Week 16	16	14 (87.5)	-2.57 (5.03)	(-13.18, 8.03)	-9.33 (10.57)	(-31.62, 12.97)	0.3897	
		Week 20	16	15 (93.8)	-0.54 (4.06)	(-9.07, 7.99)	-4.83 (8.57)	(-22.83, 13.18)	0.5801	
		Week 26	16	13 (81.3)	-3.54 (3.74)	(-11.52, 4.43)	5.13 (8.10)	(-12.14, 22.40)	0.5364	
	SoC	Week 4	8	7 (87.5)	-9.32 (6.08)	(-22.09, 3.46)				
		Week 8	8	7 (87.5)	13.15 (7.01)	(-1.65, 27.95)				
		Week 12	8	7 (87.5)	2.07 (9.63)	(-18.24, 22.38)				
		Week 16	8	7 (87.5)	6.75 (8.47)	(-11.12, 24.62)				
		Week 20	8	7 (87.5)	4.29 (7.10)	(-10.62, 19.20)				
		Week 26	8	6 (75.0)	-8.67 (6.57)	(-22.68, 5.34)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SIHD: Change from baseline in QLQ-C30 Constipation (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Constipation Male (censored)	Baseline		Peg	19	19 (100.0)	10.53 (15.92)	0.0	0.00	33.3	
			SoC	10	9 (90.0)	14.81 (17.57)	0.0	0.00	33.3	
	Week 4		Peg	19	16 (84.2)	8.33 (14.91)	0.0	0.00	33.3	
			SoC	10	9 (90.0)	18.52 (17.57)	0.0	33.33	33.3	
	Week 8		Peg	19	15 (78.9)	11.11 (16.27)	0.0	0.00	33.3	
			SoC	10	9 (90.0)	14.81 (17.57)	0.0	0.00	33.3	
	Week 12		Peg	19	17 (89.5)	9.80 (15.66)	0.0	0.00	33.3	
			SoC	10	9 (90.0)	7.41 (14.70)	0.0	0.00	33.3	
	Week 16		Peg	19	16 (84.2)	16.67 (21.08)	0.0	0.00	66.7	
			SoC	10	10 (100.0)	10.00 (16.10)	0.0	0.00	33.3	
	Week 20		Peg	19	17 (89.5)	13.73 (23.74)	0.0	0.00	66.7	
			SoC	10	9 (90.0)	14.81 (17.57)	0.0	0.00	33.3	
	Week 26		Peg	19	14 (73.7)	11.90 (16.57)	0.0	0.00	33.3	
			SoC	10	7 (70.0)	19.05 (17.82)	0.0	33.33	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SIHD: Change from baseline in QLQ-C30 Constipation (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Constipation Female (censored)	Baseline		Peg	16	16 (100.0)	14.58 (20.97)	0.0	0.00	66.7	
			SoC	8	7 (87.5)	9.52 (25.20)	0.0	0.00	66.7	
	Week 4		Peg	16	15 (93.8)	8.89 (15.26)	0.0	0.00	33.3	
			SoC	8	8 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 8		Peg	16	14 (87.5)	7.14 (14.19)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	19.05 (26.23)	0.0	0.00	66.7	
	Week 12		Peg	16	14 (87.5)	11.90 (21.11)	0.0	0.00	66.7	
			SoC	8	7 (87.5)	9.52 (25.20)	0.0	0.00	66.7	
	Week 16		Peg	16	14 (87.5)	11.90 (16.57)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	14.29 (26.23)	0.0	0.00	66.7	
	Week 20		Peg	16	15 (93.8)	13.33 (16.90)	0.0	0.00	33.3	
			SoC	8	8 (100.0)	8.33 (23.57)	0.0	0.00	66.7	
	Week 26		Peg	16	13 (81.3)	7.69 (14.62)	0.0	0.00	33.3	
			SoC	8	6 (75.0)	0.00 (0.00)	0.0	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SIH: Change from baseline in QLQ-C30 Constipation (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Constipation (censored)	Male	Week 4	Peg	19	16 (84.2)	0.00 (21.08)	-33.3	0.00	33.3	-0.19 (0.46) [-1.11, 0.69]
			SoC	10	8 (80.0)	4.17 (21.36)	-33.3	0.00	33.3	0.10 (0.46) [-0.80, 1.02]
		Week 8	Peg	19	15 (78.9)	2.22 (19.79)	-33.3	0.00	33.3	0.10 (0.46) [-0.80, 1.02]
			SoC	10	8 (80.0)	0.00 (25.20)	-33.3	0.00	33.3	0.38 (0.45) [-0.48, 1.31]
		Week 12	Peg	19	17 (89.5)	0.00 (20.41)	-33.3	0.00	33.3	0.38 (0.45) [-0.48, 1.31]
			SoC	10	8 (80.0)	-8.33 (23.57)	-33.3	0.00	33.3	0.31 (0.44) [-0.53, 1.21]
		Week 16	Peg	19	16 (84.2)	4.17 (23.96)	-33.3	0.00	66.7	0.31 (0.44) [-0.53, 1.21]
			SoC	10	9 (90.0)	-3.70 (26.06)	-33.3	0.00	33.3	0.07 (0.45) [-0.81, 0.96]
		Week 20	Peg	19	17 (89.5)	1.96 (24.92)	-33.3	0.00	66.7	0.07 (0.45) [-0.81, 0.96]
			SoC	10	8 (80.0)	0.00 (30.86)	-33.3	0.00	33.3	-0.23 (0.52) [-1.29, 0.77]
		Week 26	Peg	19	14 (73.7)	0.00 (22.65)	-33.3	0.00	33.3	-0.23 (0.52) [-1.29, 0.77]
			SoC	10	6 (60.0)	5.56 (25.09)	-33.3	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SIHD: Change from baseline in QLQ-C30 Constipation (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Constipation (censored)	Female	Week 4	Peg	16	15 (93.8)	-4.44 (24.77)	-33.3	0.00	33.3	0.20 (0.48) [-0.74, 1.18]
			SoC	8	7 (87.5)	-9.52 (25.20)	-66.7	0.00	0.0	
		Week 8	Peg	16	14 (87.5)	-7.14 (19.30)	-33.3	0.00	33.3	-0.87 (0.51) [-1.99, 0.04]
			SoC	8	7 (87.5)	9.52 (16.27)	0.0	0.00	33.3	
		Week 12	Peg	16	14 (87.5)	-2.38 (27.62)	-66.7	0.00	33.3	-0.10 (0.49) [-1.08, 0.86]
			SoC	8	7 (87.5)	0.00 (0.00)	0.0	0.00	0.0	
		Week 16	Peg	16	14 (87.5)	-2.38 (24.33)	-33.3	0.00	33.3	-0.32 (0.49) [-1.34, 0.61]
			SoC	8	7 (87.5)	4.76 (12.60)	0.0	0.00	33.3	
		Week 20	Peg	16	15 (93.8)	0.00 (21.82)	-33.3	0.00	33.3	NE (NE) [NE, NE]
			SoC	8	7 (87.5)	0.00 (0.00)	0.0	0.00	0.0	
		Week 26	Peg	16	13 (81.3)	-7.69 (24.17)	-66.7	0.00	33.3	-0.36 (0.53) [-1.46, 0.64]
			SoC	8	6 (75.0)	0.00 (0.00)	0.0	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SICB: Change from baseline in QLQ-C30 Constipation (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.1043

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SICB: Change from baseline in QLQ-C30 Constipation (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Latin America	Peg	Week 4	12	11 (91.7)	-3.46 (5.81)	(-16.60, 9.68)	-4.84 (14.77)	(-38.25, 28.57)	0.7505	
		Week 8	12	10 (83.3)	-6.67 (5.76)	(-19.94, 6.61)	-6.67 (14.10)	(-39.18, 25.85)	0.6490	
		Week 12	12	11 (91.7)	-11.95 (3.32)	(-19.45, -4.45)	-13.41 (8.43)	(-32.48, 5.66)	0.1462	
		Week 16	12	10 (83.3)	-6.67 (5.76)	(-19.94, 6.61)	-6.67 (14.10)	(-39.18, 25.85)	0.6490	
		Week 20	12	10 (83.3)	-10.00 (4.77)	(-20.99, 0.99)	-10.00 (11.68)	(-36.92, 16.92)	0.4166	
		Week 26	12	9 (75.0)	-17.85 (2.60)	(-23.99, -11.71)	-16.22 (6.07)	(-30.56, -1.87)	0.0318	*
	SoC	Week 4	2	2 (100.0)	1.38 (13.57)	(-29.32, 32.09)				
		Week 8	2	2 (100.0)	-0.00 (12.87)	(-29.68, 29.68)				
		Week 12	2	2 (100.0)	1.46 (7.75)	(-16.07, 18.99)				
		Week 16	2	2 (100.0)	-0.00 (12.87)	(-29.68, 29.68)				
		Week 20	2	2 (100.0)	-0.00 (10.66)	(-24.58, 24.58)				
		Week 26	2	2 (100.0)	-1.64 (5.48)	(-14.60, 11.33)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SICB: Change from baseline in QLQ-C30 Constipation (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Asia Pacific Peg	Peg	Week 4	23	20 (87.0)	-2.82 (3.35)	(-9.67, 4.03)	-1.91 (5.53)	(-13.22, 9.39)	0.7320	
		Week 8	23	19 (82.6)	0.62 (3.71)	(-6.98, 8.22)	-3.60 (6.03)	(-15.95, 8.75)	0.5551	
		Week 12	23	20 (87.0)	4.73 (3.76)	(-2.97, 12.42)	9.47 (6.18)	(-3.17, 22.11)	0.1362	
		Week 16	23	20 (87.0)	5.27 (4.51)	(-3.95, 14.48)	5.65 (7.15)	(-8.95, 20.26)	0.4356	
		Week 20	23	22 (95.7)	5.31 (4.54)	(-3.95, 14.58)	3.92 (7.68)	(-11.74, 19.58)	0.6131	
	Week 26	23	18 (78.3)	4.57 (3.69)	(-3.04, 12.18)	2.98 (6.57)	(-10.57, 16.54)	0.6538		
	SoC	Week 4	16	13 (81.3)	-0.91 (4.24)	(-9.58, 7.76)				
		Week 8	16	13 (81.3)	4.22 (4.54)	(-5.07, 13.51)				
		Week 12	16	13 (81.3)	-4.75 (4.75)	(-14.46, 4.97)				
		Week 16	16	14 (87.5)	-0.38 (5.42)	(-11.45, 10.69)				
Week 20		16	13 (81.3)	1.39 (6.02)	(-10.88, 13.67)					
Week 26	16	10 (62.5)	1.59 (5.28)	(-9.32, 12.49)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SIHB: Change from baseline in QLQ-C30 Constipation (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Constipation (censored)	Latin America	Baseline	Peg	12	12 (100.0)	16.67 (22.47)	0.0	0.00	66.7	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 4	Peg	12	11 (91.7)	12.12 (16.82)	0.0	0.00	33.3	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 8	Peg	12	10 (83.3)	10.00 (16.10)	0.0	0.00	33.3	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 12	Peg	12	11 (91.7)	3.03 (10.05)	0.0	0.00	33.3	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 16	Peg	12	10 (83.3)	10.00 (16.10)	0.0	0.00	33.3	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 20	Peg	12	10 (83.3)	6.67 (14.05)	0.0	0.00	33.3	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	
		Week 26	Peg	12	9 (75.0)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SIHB: Change from baseline in QLQ-C30 Constipation (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Constipation (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	10.14 (15.68)	0.0	0.00	33.3	
			SoC	16	14 (87.5)	11.90 (21.11)	0.0	0.00	66.7	
	Week 4	Peg	23	20 (87.0)	6.67 (13.68)	0.0	0.00	33.3		
		SoC	16	15 (93.8)	8.89 (15.26)	0.0	0.00	33.3		
	Week 8	Peg	23	19 (82.6)	8.77 (15.08)	0.0	0.00	33.3		
		SoC	16	14 (87.5)	16.67 (21.68)	0.0	0.00	66.7		
	Week 12	Peg	23	20 (87.0)	15.00 (20.16)	0.0	0.00	66.7		
		SoC	16	14 (87.5)	7.14 (19.30)	0.0	0.00	66.7		
	Week 16	Peg	23	20 (87.0)	16.67 (20.23)	0.0	0.00	66.7		
		SoC	16	15 (93.8)	11.11 (20.57)	0.0	0.00	66.7		
	Week 20	Peg	23	22 (95.7)	16.67 (22.42)	0.0	0.00	66.7		
		SoC	16	15 (93.8)	11.11 (20.57)	0.0	0.00	66.7		
	Week 26	Peg	23	18 (78.3)	14.81 (17.04)	0.0	0.00	33.3		
		SoC	16	11 (68.8)	9.09 (15.57)	0.0	0.00	33.3		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SIHB: Change from baseline in QLQ-C30 Constipation (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Constipation (censored)	Latin America	Week 4	Peg	12	11 (91.7)	-3.03 (27.71)	-33.3	0.00	33.3	-0.09 (0.85) [-1.81, 1.58]
			SoC	2	2 (100.0)	0.00 (47.14)	-33.3	0.00	33.3	
		Week 8	Peg	12	10 (83.3)	-6.67 (21.08)	-33.3	0.00	33.3	-0.25 (0.87) [-2.04, 1.42]
			SoC	2	2 (100.0)	0.00 (47.14)	-33.3	0.00	33.3	
		Week 12	Peg	12	11 (91.7)	-12.12 (26.97)	-66.7	0.00	33.3	-0.38 (0.86) [-2.17, 1.23]
			SoC	2	2 (100.0)	0.00 (47.14)	-33.3	0.00	33.3	
		Week 16	Peg	12	10 (83.3)	-6.67 (21.08)	-33.3	0.00	33.3	-0.25 (0.87) [-2.04, 1.42]
			SoC	2	2 (100.0)	0.00 (47.14)	-33.3	0.00	33.3	
		Week 20	Peg	12	10 (83.3)	-10.00 (16.10)	-33.3	0.00	0.0	-0.43 (0.87) [-2.28, 1.20]
			SoC	2	2 (100.0)	0.00 (47.14)	-33.3	0.00	33.3	
		Week 26	Peg	12	9 (75.0)	-18.52 (24.22)	-66.7	0.00	0.0	-0.61 (0.91) [-2.59, 1.01]
			SoC	2	2 (100.0)	0.00 (47.14)	-33.3	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SIHB: Change from baseline in QLQ-C30 Constipation (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline in QLQ-C30 Constipation (censored)	Asia Pacific	Week 4	Peg	23	20 (87.0)	-1.67 (20.16)	-33.3	0.00	33.3	0.04	(0.37) [-0.68, 0.77]
			SoC	16	13 (81.3)	-2.56 (21.35)	-66.7	0.00	33.3		
		Week 8	Peg	23	19 (82.6)	0.00 (19.25)	-33.3	0.00	33.3	-0.26	(0.37) [-1.02, 0.45]
			SoC	16	13 (81.3)	5.13 (18.49)	-33.3	0.00	33.3		
		Week 12	Peg	23	20 (87.0)	5.00 (19.57)	-33.3	0.00	33.3	0.57	(0.38) [-0.13, 1.36]
			SoC	16	13 (81.3)	-5.13 (12.52)	-33.3	0.00	0.0		
		Week 16	Peg	23	20 (87.0)	5.00 (24.84)	-33.3	0.00	66.7	0.22	(0.36) [-0.48, 0.94]
			SoC	16	14 (87.5)	0.00 (18.49)	-33.3	0.00	33.3		
		Week 20	Peg	23	22 (95.7)	6.06 (24.42)	-33.3	0.00	66.7	0.26	(0.36) [-0.44, 0.99]
			SoC	16	13 (81.3)	0.00 (19.25)	-33.3	0.00	33.3		
		Week 26	Peg	23	18 (78.3)	3.70 (19.43)	-33.3	0.00	33.3	0.02	(0.41) [-0.79, 0.83]
			SoC	16	10 (62.5)	3.33 (10.54)	0.0	0.00	33.3		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SICA: Change from baseline in QLQ-C30 Constipation (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.8514

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SICA: Change from baseline in QLQ-C30 Constipation (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 4	21	18 (85.7)	-5.63 (3.62)	(-13.19, 1.93)	-5.89 (7.77)	(-22.11, 10.32)	0.4571	
		Week 8	21	17 (81.0)	-5.82 (3.39)	(-12.91, 1.28)	1.07 (7.11)	(-13.81, 15.95)	0.8817	
		Week 12	21	18 (85.7)	-1.91 (4.17)	(-10.61, 6.78)	11.19 (8.94)	(-7.45, 29.84)	0.2250	
		Week 16	21	17 (81.0)	0.89 (3.88)	(-7.20, 8.98)	8.98 (7.63)	(-6.92, 24.89)	0.2527	
		Week 20	21	18 (85.7)	2.08 (4.67)	(-7.66, 11.82)	2.91 (10.02)	(-18.00, 23.81)	0.7747	
	Week 26	21	15 (71.4)	-6.02 (4.16)	(-14.88, 2.85)	-2.78 (10.22)	(-24.56, 19.00)	0.7895		
	SoC	Week 4	8	5 (62.5)	0.26 (6.88)	(-14.08, 14.61)				
		Week 8	8	5 (62.5)	-6.89 (6.25)	(-19.97, 6.19)				
		Week 12	8	5 (62.5)	-13.11 (7.91)	(-29.60, 3.39)				
		Week 16	8	6 (75.0)	-8.09 (6.55)	(-21.74, 5.57)				
		Week 20	8	5 (62.5)	-0.83 (8.86)	(-19.32, 17.67)				
Week 26	8	3 (37.5)	-3.24 (9.32)	(-23.11, 16.63)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SICA: Change from baseline in QLQ-C30 Constipation (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	0.43 (4.53)	(-9.02, 9.88)	0.99 (6.92)	(-13.44, 15.42)	0.8872	
		Week 8	14	12 (85.7)	1.60 (5.07)	(-9.01, 12.21)	-9.82 (7.55)	(-25.63, 6.00)	0.2094	
		Week 12	14	13 (92.9)	-0.75 (4.97)	(-11.12, 9.63)	-1.72 (7.55)	(-17.48, 14.04)	0.8225	
		Week 16	14	13 (92.9)	1.69 (6.12)	(-11.08, 14.45)	-2.79 (9.30)	(-22.18, 16.60)	0.7672	
		Week 20	14	14 (100.0)	-0.87 (5.44)	(-12.19, 10.44)	-2.10 (8.45)	(-19.68, 15.48)	0.8062	
		Week 26	14	12 (85.7)	1.21 (4.79)	(-8.85, 11.27)	-0.88 (7.35)	(-16.31, 14.56)	0.9063	
	SoC	Week 4	10	10 (100.0)	-0.56 (5.17)	(-11.36, 10.23)				
		Week 8	10	10 (100.0)	11.41 (5.56)	(-0.21, 23.04)				
		Week 12	10	10 (100.0)	0.97 (5.67)	(-10.86, 12.80)				
		Week 16	10	10 (100.0)	4.48 (6.98)	(-10.08, 19.03)				
		Week 20	10	10 (100.0)	1.22 (6.44)	(-12.17, 14.62)				
		Week 26	10	9 (90.0)	2.09 (5.54)	(-9.54, 13.72)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SIHA: Change from baseline in QLQ-C30 Constipation (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Constipation < 4 (censored)	Baseline	Peg	21	21 (100.0)	14.29 (19.92)	0.0	0.00	66.7	
		SoC	8	6 (75.0)	11.11 (17.21)	0.0	0.00	33.3	
	Week 4	Peg	21	18 (85.7)	7.41 (14.26)	0.0	0.00	33.3	
		SoC	8	7 (87.5)	9.52 (16.27)	0.0	0.00	33.3	
	Week 8	Peg	21	17 (81.0)	7.84 (14.57)	0.0	0.00	33.3	
		SoC	8	6 (75.0)	5.56 (13.61)	0.0	0.00	33.3	
	Week 12	Peg	21	18 (85.7)	11.11 (19.80)	0.0	0.00	66.7	
		SoC	8	6 (75.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 16	Peg	21	17 (81.0)	15.69 (17.15)	0.0	0.00	33.3	
		SoC	8	7 (87.5)	4.76 (12.60)	0.0	0.00	33.3	
	Week 20	Peg	21	18 (85.7)	16.67 (20.61)	0.0	0.00	66.7	
		SoC	8	7 (87.5)	9.52 (16.27)	0.0	0.00	33.3	
	Week 26	Peg	21	15 (71.4)	8.89 (15.26)	0.0	0.00	33.3	
		SoC	8	4 (50.0)	8.33 (16.67)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SIHA: Change from baseline in QLQ-C30 Constipation (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Constipation >= 4 (censored)	Baseline	Peg	14	14 (100.0)	9.52 (15.63)	0.0	0.00	33.3	
		SoC	10	10 (100.0)	13.33 (23.31)	0.0	0.00	66.7	
	Week 4	Peg	14	13 (92.9)	10.26 (16.01)	0.0	0.00	33.3	
		SoC	10	10 (100.0)	10.00 (16.10)	0.0	0.00	33.3	
	Week 8	Peg	14	12 (85.7)	11.11 (16.41)	0.0	0.00	33.3	
		SoC	10	10 (100.0)	23.33 (22.50)	0.0	33.33	66.7	
	Week 12	Peg	14	13 (92.9)	10.26 (16.01)	0.0	0.00	33.3	
		SoC	10	10 (100.0)	13.33 (23.31)	0.0	0.00	66.7	
	Week 16	Peg	14	13 (92.9)	12.82 (21.68)	0.0	0.00	66.7	
		SoC	10	10 (100.0)	16.67 (23.57)	0.0	0.00	66.7	
	Week 20	Peg	14	14 (100.0)	9.52 (20.37)	0.0	0.00	66.7	
		SoC	10	10 (100.0)	13.33 (23.31)	0.0	0.00	66.7	
	Week 26	Peg	14	12 (85.7)	11.11 (16.41)	0.0	0.00	33.3	
		SoC	10	9 (90.0)	11.11 (16.67)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SIHA: Change from baseline in QLQ-C30 Constipation (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in QLQ-C30 Constipation (censored)	Week 4	Peg	21	18 (85.7)	-5.56 (23.57)	-33.3	0.00	33.3	-0.23 (0.53) [-1.31, 0.80]
		SoC	8	5 (62.5)	0.00 (23.57)	-33.3	0.00	33.3	
	Week 8	Peg	21	17 (81.0)	-5.88 (17.62)	-33.3	0.00	33.3	0.04 (0.54) [-1.02, 1.10]
		SoC	8	5 (62.5)	-6.67 (27.89)	-33.3	0.00	33.3	
	Week 12	Peg	21	18 (85.7)	-1.85 (24.18)	-66.7	0.00	33.3	0.48 (0.54) [-0.53, 1.59]
		SoC	8	5 (62.5)	-13.33 (18.26)	-33.3	0.00	0.0	
	Week 16	Peg	21	17 (81.0)	0.00 (20.41)	-33.3	0.00	33.3	0.25 (0.50) [-0.72, 1.27]
		SoC	8	6 (75.0)	-5.56 (25.09)	-33.3	0.00	33.3	
	Week 20	Peg	21	18 (85.7)	1.85 (21.30)	-33.3	0.00	33.3	0.07 (0.53) [-0.97, 1.13]
		SoC	8	5 (62.5)	0.00 (33.33)	-33.3	0.00	33.3	
	Week 26	Peg	21	15 (71.4)	-6.67 (22.54)	-66.7	0.00	33.3	-0.26 (0.68) [-1.65, 1.04]
		SoC	8	3 (37.5)	0.00 (33.33)	-33.3	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QCWC_SIHA: Change from baseline in QLQ-C30 Constipation (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in QLQ-C30 Constipation (censored)	Week 4	Peg	14	13 (92.9)	2.56 (21.35)	-33.3	0.00	33.3	0.25 (0.44) [-0.60, 1.16]
		SoC	10	10 (100.0)	-3.33 (24.60)	-66.7	0.00	33.3	
	Week 8	Peg	14	12 (85.7)	2.78 (22.29)	-33.3	0.00	33.3	-0.35 (0.46) [-1.29, 0.51]
		SoC	10	10 (100.0)	10.00 (16.10)	0.0	0.00	33.3	
	Week 12	Peg	14	13 (92.9)	0.00 (23.57)	-33.3	0.00	33.3	NE (NE) [NE, NE]
		SoC	10	10 (100.0)	0.00 (15.71)	-33.3	0.00	33.3	
	Week 16	Peg	14	13 (92.9)	2.56 (28.74)	-33.3	0.00	66.7	-0.03 (0.44) [-0.91, 0.84]
		SoC	10	10 (100.0)	3.33 (18.92)	-33.3	0.00	33.3	
	Week 20	Peg	14	14 (100.0)	0.00 (26.15)	-33.3	0.00	66.7	NE (NE) [NE, NE]
		SoC	10	10 (100.0)	0.00 (15.71)	-33.3	0.00	33.3	
	Week 26	Peg	14	12 (85.7)	0.00 (24.62)	-33.3	0.00	33.3	-0.18 (0.47) [-1.12, 0.73]
		SoC	10	9 (90.0)	3.70 (11.11)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SICC: Change from baseline in QLQ-C30 Diarrhoea (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.7569

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SICC: Change from baseline in QLQ-C30 Diarrhoea (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 60	Peg	Week 4	29	26 (89.7)	-5.36 (2.15)	(-9.73, -0.98)	0.33 (4.04)	(-7.89, 8.55)	0.9356	
		Week 8	29	24 (82.8)	-2.19 (2.94)	(-8.19, 3.82)	-3.97 (5.39)	(-14.96, 7.01)	0.4662	
		Week 12	29	26 (89.7)	-4.09 (2.32)	(-8.81, 0.63)	1.63 (4.37)	(-7.25, 10.51)	0.7114	
		Week 16	29	25 (86.2)	-0.61 (3.20)	(-7.11, 5.90)	-2.18 (5.73)	(-13.83, 9.48)	0.7065	
		Week 20	29	27 (93.1)	-0.23 (2.79)	(-5.89, 5.43)	-0.79 (5.30)	(-11.57, 9.99)	0.8822	
		Week 26	29	23 (79.3)	-5.88 (2.66)	(-11.33, -0.44)	-2.65 (5.03)	(-12.94, 7.63)	0.6016	
	SoC	Week 4	13	11 (84.6)	-5.69 (3.34)	(-12.49, 1.12)				
		Week 8	13	11 (84.6)	1.79 (4.39)	(-7.18, 10.75)				
		Week 12	13	11 (84.6)	-5.72 (3.61)	(-13.07, 1.62)				
		Week 16	13	12 (92.3)	1.57 (4.65)	(-7.90, 11.04)				
		Week 20	13	11 (84.6)	0.56 (4.43)	(-8.44, 9.56)				
		Week 26	13	10 (76.9)	-3.23 (4.15)	(-11.71, 5.25)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SICC: Change from baseline in QLQ-C30 Diarrhoea (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	7.69 (4.83)	(-4.14, 19.52)	10.26 (7.64)	(-8.45, 28.96)	0.2283	
		Week 8	6	5 (83.3)	0.00 (NE)	(NE, NE)	NE (NE)	(NE, NE)	NE	
		Week 12	6	5 (83.3)	7.69 (4.83)	(-4.14, 19.52)	10.26 (7.64)	(-8.45, 28.96)	0.2283	
		Week 16	6	5 (83.3)	7.69 (4.83)	(-4.14, 19.52)	10.26 (7.64)	(-8.45, 28.96)	0.2283	
		Week 20	6	5 (83.3)	0.00 (NE)	(NE, NE)	NE (NE)	(NE, NE)	NE	
		Week 26	6	4 (66.7)	0.00 (NE)	(NE, NE)	NE (NE)	(NE, NE)	NE	
	SoC	Week 4	5	4 (80.0)	-2.56 (5.67)	(-16.44, 11.31)				
		Week 8	5	4 (80.0)	0.00 (NE)	(NE, NE)				
		Week 12	5	4 (80.0)	-2.56 (5.67)	(-16.44, 11.31)				
		Week 16	5	4 (80.0)	-2.56 (5.67)	(-16.44, 11.31)				
		Week 20	5	4 (80.0)	0.00 (NE)	(NE, NE)				
		Week 26	5	2 (40.0)	0.00 (NE)	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SIHC: Change from baseline in QLQ-C30 Diarrhoea (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Diarrhoea (censored)	< 60	Baseline	Peg	29	29 (100.0)	10.34 (18.05)	0.0	0.00	66.7	
			SoC	13	12 (92.3)	2.78 (9.62)	0.0	0.00	33.3	
		Week 4	Peg	29	26 (89.7)	3.85 (10.86)	0.0	0.00	33.3	
			SoC	13	12 (92.3)	2.78 (9.62)	0.0	0.00	33.3	
		Week 8	Peg	29	24 (82.8)	6.94 (13.83)	0.0	0.00	33.3	
			SoC	13	11 (84.6)	9.09 (15.57)	0.0	0.00	33.3	
		Week 12	Peg	29	26 (89.7)	5.13 (12.26)	0.0	0.00	33.3	
			SoC	13	11 (84.6)	3.03 (10.05)	0.0	0.00	33.3	
		Week 16	Peg	29	25 (86.2)	8.00 (17.43)	0.0	0.00	66.7	
			SoC	13	12 (92.3)	8.33 (15.08)	0.0	0.00	33.3	
		Week 20	Peg	29	27 (93.1)	9.88 (18.06)	0.0	0.00	66.7	
			SoC	13	12 (92.3)	5.56 (12.97)	0.0	0.00	33.3	
		Week 26	Peg	29	23 (79.3)	4.35 (11.48)	0.0	0.00	33.3	
			SoC	13	10 (76.9)	6.67 (14.05)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SIHC: Change from baseline in QLQ-C30 Diarrhoea (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Diarrhoea (censored)	>= 60	Baseline	Peg	6	6 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	5	4 (80.0)	0.00 (0.00)	0.0	0.00	0.0	
		Week 4	Peg	6	5 (83.3)	6.67 (14.91)	0.0	0.00	33.3	
			SoC	5	5 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
		Week 8	Peg	6	5 (83.3)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	5	5 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
		Week 12	Peg	6	5 (83.3)	6.67 (14.91)	0.0	0.00	33.3	
			SoC	5	5 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
		Week 16	Peg	6	5 (83.3)	6.67 (14.91)	0.0	0.00	33.3	
			SoC	5	5 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
		Week 20	Peg	6	5 (83.3)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	5	5 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
		Week 26	Peg	6	4 (66.7)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	5	3 (60.0)	0.00 (0.00)	0.0	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SIHC: Change from baseline in QLQ-C30 Diarrhoea (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in QLQ-C30 Diarrhoea (censored)	Week 4	Peg	29	26 (89.7)	-7.69 (21.72)	-66.7	0.00	33.3	-0.38 (0.37) [-1.14, 0.34]
		SoC	13	11 (84.6)	0.00 (14.91)	-33.3	0.00	33.3	
	Week 8	Peg	29	24 (82.8)	-4.17 (17.89)	-33.3	0.00	33.3	-0.54 (0.38) [-1.33, 0.18]
		SoC	13	11 (84.6)	6.06 (20.10)	-33.3	0.00	33.3	
	Week 12	Peg	29	26 (89.7)	-6.41 (23.13)	-66.7	0.00	33.3	-0.30 (0.37) [-1.05, 0.42]
		SoC	13	11 (84.6)	0.00 (14.91)	-33.3	0.00	33.3	
	Week 16	Peg	29	25 (86.2)	-2.67 (21.34)	-33.3	0.00	33.3	-0.42 (0.37) [-1.17, 0.27]
		SoC	13	12 (92.3)	5.56 (12.97)	0.0	0.00	33.3	
	Week 20	Peg	29	27 (93.1)	-1.23 (17.25)	-33.3	0.00	33.3	-0.27 (0.37) [-1.01, 0.44]
		SoC	13	11 (84.6)	3.03 (10.05)	0.0	0.00	33.3	
	Week 26	Peg	29	23 (79.3)	-8.70 (22.96)	-66.7	0.00	33.3	-0.58 (0.40) [-1.41, 0.16]
		SoC	13	10 (76.9)	3.33 (10.54)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SIHC: Change from baseline in QLQ-C30 Diarrhoea (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in QLQ-C30 Diarrhoea (censored)	Week 4	Peg	6	5 (83.3)	6.67 (14.91)	0.0	0.00	33.3	0.53 (0.82) [-0.89, 2.35]
		SoC	5	4 (80.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 8	Peg	6	5 (83.3)	0.00 (0.00)	0.0	0.00	0.0	NE (NE) [NE, NE]
		SoC	5	4 (80.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 12	Peg	6	5 (83.3)	6.67 (14.91)	0.0	0.00	33.3	0.53 (0.82) [-0.89, 2.35]
		SoC	5	4 (80.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 16	Peg	6	5 (83.3)	6.67 (14.91)	0.0	0.00	33.3	0.53 (0.82) [-0.89, 2.35]
		SoC	5	4 (80.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 20	Peg	6	5 (83.3)	0.00 (0.00)	0.0	0.00	0.0	NE (NE) [NE, NE]
		SoC	5	4 (80.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 26	Peg	6	4 (66.7)	0.00 (0.00)	0.0	0.00	0.0	NE (NE) [NE, NE]
		SoC	5	2 (40.0)	0.00 (0.00)	0.0	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SICD: Change from baseline in QLQ-C30 Diarrhoea (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.7018

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SICD: Change from baseline in QLQ-C30 Diarrhoea (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Male	Peg	Week 4	19	16 (84.2)	-6.10 (2.51)	(-11.33, -0.88)	-1.66 (4.42)	(-10.87, 7.55)	0.7111	
		Week 8	19	15 (78.9)	-5.41 (2.82)	(-11.31, 0.50)	-7.68 (4.87)	(-17.87, 2.50)	0.1308	
		Week 12	19	17 (89.5)	-2.15 (3.18)	(-8.76, 4.46)	2.61 (5.75)	(-9.34, 14.57)	0.6539	
		Week 16	19	16 (84.2)	-1.20 (3.04)	(-7.52, 5.11)	-5.13 (5.23)	(-16.00, 5.73)	0.3370	
		Week 20	19	17 (89.5)	0.31 (2.36)	(-4.60, 5.22)	0.23 (4.27)	(-8.65, 9.11)	0.9572	
		Week 26	19	14 (73.7)	-7.83 (2.93)	(-14.04, -1.63)	-3.89 (5.41)	(-15.35, 7.58)	0.4828	
	SoC	Week 4	10	8 (80.0)	-4.44 (3.64)	(-12.04, 3.15)				
		Week 8	10	8 (80.0)	2.28 (3.96)	(-6.00, 10.55)				
		Week 12	10	8 (80.0)	-4.76 (4.76)	(-14.67, 5.14)				
		Week 16	10	9 (90.0)	3.93 (4.20)	(-4.80, 12.67)				
		Week 20	10	8 (80.0)	0.07 (3.54)	(-7.29, 7.43)				
		Week 26	10	6 (60.0)	-3.95 (4.50)	(-13.49, 5.60)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SICD: Change from baseline in QLQ-C30 Diarrhoea (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Female	Peg	Week 4	16	15 (93.8)	-0.06 (3.32)	(-7.04, 6.92)	3.62 (7.76)	(-12.69, 19.93)	0.6468	
		Week 8	16	14 (87.5)	2.50 (4.53)	(-7.05, 12.05)	3.04 (10.78)	(-19.71, 25.79)	0.7814	
		Week 12	16	14 (87.5)	0.71 (2.93)	(-5.47, 6.89)	13.48 (6.98)	(-1.25, 28.20)	0.0704	
		Week 16	16	14 (87.5)	5.27 (5.42)	(-6.16, 16.70)	14.39 (12.90)	(-12.83, 41.60)	0.2803	
		Week 20	16	15 (93.8)	1.54 (3.79)	(-6.43, 9.51)	7.12 (8.87)	(-11.50, 25.75)	0.4321	
		Week 26	16	13 (81.3)	-2.27 (4.11)	(-11.04, 6.49)	-1.54 (9.95)	(-22.74, 19.66)	0.8790	
	SoC	Week 4	8	7 (87.5)	-3.68 (6.29)	(-16.88, 9.53)				
		Week 8	8	7 (87.5)	-0.54 (8.38)	(-18.22, 17.14)				
		Week 12	8	7 (87.5)	-12.77 (5.42)	(-24.21, -1.32)				
		Week 16	8	7 (87.5)	-9.12 (10.03)	(-30.27, 12.03)				
		Week 20	8	7 (87.5)	-5.59 (7.18)	(-20.67, 9.50)				
		Week 26	8	6 (75.0)	-0.73 (7.94)	(-17.66, 16.20)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SIHD: Change from baseline in QLQ-C30 Diarrhoea (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Diarrhoea (censored)	Male	Baseline	Peg	19	19 (100.0)	8.77 (18.73)	0.0	0.00	66.7	
			SoC	10	9 (90.0)	3.70 (11.11)	0.0	0.00	33.3	
		Week 4	Peg	19	16 (84.2)	2.08 (8.33)	0.0	0.00	33.3	
			SoC	10	9 (90.0)	3.70 (11.11)	0.0	0.00	33.3	
		Week 8	Peg	19	15 (78.9)	2.22 (8.61)	0.0	0.00	33.3	
			SoC	10	9 (90.0)	7.41 (14.70)	0.0	0.00	33.3	
		Week 12	Peg	19	17 (89.5)	5.88 (13.10)	0.0	0.00	33.3	
			SoC	10	9 (90.0)	3.70 (11.11)	0.0	0.00	33.3	
		Week 16	Peg	19	16 (84.2)	6.25 (13.44)	0.0	0.00	33.3	
			SoC	10	10 (100.0)	6.67 (14.05)	0.0	0.00	33.3	
		Week 20	Peg	19	17 (89.5)	9.80 (19.60)	0.0	0.00	66.7	
			SoC	10	9 (90.0)	3.70 (11.11)	0.0	0.00	33.3	
		Week 26	Peg	19	14 (73.7)	2.38 (8.91)	0.0	0.00	33.3	
			SoC	10	7 (70.0)	4.76 (12.60)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SIHD: Change from baseline in QLQ-C30 Diarrhoea (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Diarrhoea (censored)	Female	Baseline	Peg	16	16 (100.0)	8.33 (14.91)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	0.00 (0.00)	0.0	0.00	0.0	
		Week 4	Peg	16	15 (93.8)	6.67 (13.80)	0.0	0.00	33.3	
			SoC	8	8 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
		Week 8	Peg	16	14 (87.5)	9.52 (15.63)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	4.76 (12.60)	0.0	0.00	33.3	
		Week 12	Peg	16	14 (87.5)	4.76 (12.10)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	0.00 (0.00)	0.0	0.00	0.0	
		Week 16	Peg	16	14 (87.5)	9.52 (20.37)	0.0	0.00	66.7	
			SoC	8	7 (87.5)	4.76 (12.60)	0.0	0.00	33.3	
		Week 20	Peg	16	15 (93.8)	6.67 (13.80)	0.0	0.00	33.3	
			SoC	8	8 (100.0)	4.17 (11.79)	0.0	0.00	33.3	
		Week 26	Peg	16	13 (81.3)	5.13 (12.52)	0.0	0.00	33.3	
			SoC	8	6 (75.0)	5.56 (13.61)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SIHD: Change from baseline in QLQ-C30 Diarrhoea (censored) by sex
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Male in QLQ-C30 Diarrhoea (censored)	Week 4	Peg	19	16 (84.2)	-8.33 (22.77)	-66.7	0.00	33.3	-0.38 (0.46) [-1.32, 0.49]
		SoC	10	8 (80.0)	0.00 (17.82)	-33.3	0.00	33.3	
	Week 8	Peg	19	15 (78.9)	-6.67 (13.80)	-33.3	0.00	0.0	-0.63 (0.47) [-1.63, 0.24]
		SoC	10	8 (80.0)	4.17 (21.36)	-33.3	0.00	33.3	
	Week 12	Peg	19	17 (89.5)	-3.92 (23.22)	-66.7	0.00	33.3	-0.17 (0.45) [-1.08, 0.70]
		SoC	10	8 (80.0)	0.00 (17.82)	-33.3	0.00	33.3	
	Week 16	Peg	19	16 (84.2)	-2.08 (19.12)	-33.3	0.00	33.3	-0.33 (0.44) [-1.24, 0.50]
		SoC	10	9 (90.0)	3.70 (11.11)	0.0	0.00	33.3	
	Week 20	Peg	19	17 (89.5)	0.00 (11.79)	-33.3	0.00	33.3	NE (NE) [NE, NE]
		SoC	10	8 (80.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 26	Peg	19	14 (73.7)	-9.52 (24.21)	-66.7	0.00	33.3	-0.44 (0.52) [-1.54, 0.54]
		SoC	10	6 (60.0)	0.00 (0.00)	0.0	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SIHD: Change from baseline in QLQ-C30 Diarrhoea (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Diarrhoea (censored)	Female	Week 4	Peg	16	15 (93.8)	-2.22 (19.79)	-33.3	0.00	33.3	-0.13 (0.48) [-1.10, 0.81]
			SoC	8	7 (87.5)	0.00 (0.00)	0.0	0.00	0.0	
		Week 8	Peg	16	14 (87.5)	0.00 (18.49)	-33.3	0.00	33.3	-0.27 (0.49) [-1.28, 0.67]
			SoC	8	7 (87.5)	4.76 (12.60)	0.0	0.00	33.3	
		Week 12	Peg	16	14 (87.5)	-4.76 (22.10)	-33.3	0.00	33.3	-0.25 (0.49) [-1.25, 0.69]
			SoC	8	7 (87.5)	0.00 (0.00)	0.0	0.00	0.0	
		Week 16	Peg	16	14 (87.5)	0.00 (22.65)	-33.3	0.00	33.3	-0.23 (0.49) [-1.23, 0.72]
			SoC	8	7 (87.5)	4.76 (12.60)	0.0	0.00	33.3	
		Week 20	Peg	16	15 (93.8)	-2.22 (19.79)	-33.3	0.00	33.3	-0.37 (0.49) [-1.38, 0.54]
			SoC	8	7 (87.5)	4.76 (12.60)	0.0	0.00	33.3	
		Week 26	Peg	16	13 (81.3)	-5.13 (18.49)	-33.3	0.00	33.3	-0.59 (0.54) [-1.74, 0.39]
			SoC	8	6 (75.0)	5.56 (13.61)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SICB: Change from baseline in QLQ-C30 Diarrhoea (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.7123

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SICB: Change from baseline in QLQ-C30 Diarrhoea (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Latin America	Peg	Week 4	12	11 (91.7)	-12.41 (2.92)	(-19.01, -5.81)	1.03 (7.75)	(-16.51, 18.56)	0.8973	
		Week 8	12	10 (83.3)	-5.51 (2.67)	(-11.67, 0.65)	0.25 (6.79)	(-15.41, 15.91)	0.9714	
		Week 12	12	11 (91.7)	-15.38 (NE)	(NE, NE)	NE (NE)	(NE, NE)	NE	
		Week 16	12	10 (83.3)	-1.92 (6.51)	(-16.93, 13.09)	5.16 (16.55)	(-33.01, 43.32)	0.7633	
		Week 20	12	10 (83.3)	-8.88 (4.71)	(-19.74, 1.98)	-3.28 (12.20)	(-31.42, 24.86)	0.7950	
		Week 26	12	9 (75.0)	-14.99 (3.83)	(-24.05, -5.93)	2.13 (9.60)	(-20.58, 24.84)	0.8310	
	SoC	Week 4	2	2 (100.0)	-13.44 (7.09)	(-29.47, 2.59)				
		Week 8	2	2 (100.0)	-5.77 (6.15)	(-19.96, 8.43)				
		Week 12	2	2 (100.0)	-15.38 (NE)	(NE, NE)				
		Week 16	2	2 (100.0)	-7.08 (15.00)	(-41.67, 27.51)				
		Week 20	2	2 (100.0)	-5.60 (11.02)	(-31.01, 19.81)				
		Week 26	2	2 (100.0)	-17.12 (8.58)	(-37.41, 3.17)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SICB: Change from baseline in QLQ-C30 Diarrhoea (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Asia Pacific Peg	Peg	Week 4	23	20 (87.0)	0.67 (2.53)	(-4.50, 5.85)	1.53 (4.15)	(-6.97, 10.03)	0.7156	
		Week 8	23	19 (82.6)	-1.04 (2.92)	(-7.01, 4.93)	-5.12 (4.72)	(-14.80, 4.55)	0.2876	
		Week 12	23	20 (87.0)	3.97 (3.01)	(-2.19, 10.13)	4.84 (4.95)	(-5.28, 14.96)	0.3357	
		Week 16	23	20 (87.0)	1.35 (3.01)	(-4.80, 7.50)	-1.49 (4.78)	(-11.25, 8.28)	0.7581	
		Week 20	23	22 (95.7)	3.72 (2.60)	(-1.58, 9.03)	2.52 (4.39)	(-6.43, 11.47)	0.5702	
	Week 26	23	18 (78.3)	-0.98 (2.90)	(-6.96, 5.00)	-1.96 (5.14)	(-12.57, 8.65)	0.7063		
	SoC	Week 4	16	13 (81.3)	-0.86 (3.19)	(-7.39, 5.68)				
		Week 8	16	13 (81.3)	4.08 (3.56)	(-3.21, 11.38)				
		Week 12	16	13 (81.3)	-0.87 (3.80)	(-8.65, 6.91)				
		Week 16	16	14 (87.5)	2.83 (3.62)	(-4.55, 10.22)				
Week 20		16	13 (81.3)	1.20 (3.44)	(-5.82, 8.23)					
Week 26	16	10 (62.5)	0.98 (4.15)	(-7.58, 9.54)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SIHb: Change from baseline in QLQ-C30 Diarrhoea (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE)
										[95% CI]
QLQ-C30 Diarrhoea (censored)	Latin America	Baseline	Peg	12	12 (100.0)	16.67 (22.47)	0.0	0.00	66.7	
			SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
		Week 4	Peg	12	11 (91.7)	3.03 (10.05)	0.0	0.00	33.3	
			SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
		Week 8	Peg	12	10 (83.3)	10.00 (16.10)	0.0	0.00	33.3	
			SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
		Week 12	Peg	12	11 (91.7)	0.00 (0.00)	0.0	0.00	0.0	
			SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
		Week 16	Peg	12	10 (83.3)	13.33 (23.31)	0.0	0.00	66.7	
			SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
		Week 20	Peg	12	10 (83.3)	10.00 (22.50)	0.0	0.00	66.7	
			SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
		Week 26	Peg	12	9 (75.0)	3.70 (11.11)	0.0	0.00	33.3	
			SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SIHB: Change from baseline in QLQ-C30 Diarrhoea (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Diarrhoea (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	4.35 (11.48)	0.0	0.00	33.3	
			SoC	16	14 (87.5)	2.38 (8.91)	0.0	0.00	33.3	
		Week 4	Peg	23	20 (87.0)	5.00 (12.21)	0.0	0.00	33.3	
			SoC	16	15 (93.8)	2.22 (8.61)	0.0	0.00	33.3	
		Week 8	Peg	23	19 (82.6)	3.51 (10.51)	0.0	0.00	33.3	
			SoC	16	14 (87.5)	7.14 (14.19)	0.0	0.00	33.3	
		Week 12	Peg	23	20 (87.0)	8.33 (14.81)	0.0	0.00	33.3	
			SoC	16	14 (87.5)	2.38 (8.91)	0.0	0.00	33.3	
		Week 16	Peg	23	20 (87.0)	5.00 (12.21)	0.0	0.00	33.3	
			SoC	16	15 (93.8)	6.67 (13.80)	0.0	0.00	33.3	
		Week 20	Peg	23	22 (95.7)	7.58 (14.30)	0.0	0.00	33.3	
			SoC	16	15 (93.8)	4.44 (11.73)	0.0	0.00	33.3	
		Week 26	Peg	23	18 (78.3)	3.70 (10.78)	0.0	0.00	33.3	
			SoC	16	11 (68.8)	6.06 (13.48)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SIHB: Change from baseline in QLQ-C30 Diarrhoea (censored) by region
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Latin in QLQ-C30 Diarrhoea America (censored)	Week 4	Peg	12	11 (91.7)	-15.15 (22.92)	-66.7	0.00	0.0	-0.65 (0.87) [-2.51, 0.93]
		SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 8	Peg	12	10 (83.3)	-6.67 (14.05)	-33.3	0.00	0.0	-0.46 (0.88) [-2.32, 1.16]
		SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 12	Peg	12	11 (91.7)	-18.18 (22.92)	-66.7	0.00	0.0	-0.77 (0.87) [-2.68, 0.78]
		SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 16	Peg	12	10 (83.3)	-3.33 (24.60)	-33.3	0.00	33.3	-0.13 (0.87) [-1.89, 1.56]
		SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 20	Peg	12	10 (83.3)	-10.00 (16.10)	-33.3	0.00	0.0	-0.60 (0.88) [-2.51, 1.00]
		SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 26	Peg	12	9 (75.0)	-18.52 (24.22)	-66.7	0.00	0.0	-0.74 (0.91) [-2.77, 0.86]
		SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.

95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.

Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SIHB: Change from baseline in QLQ-C30 Diarrhoea (censored) by region
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Asia in QLQ-C30 Diarrhoea Pacific (censored)	Week 4	Peg	23	20 (87.0)	0.00 (18.73)	-33.3	0.00	33.3	NE (NE) [NE, NE]
		SoC	16	13 (81.3)	0.00 (13.61)	-33.3	0.00	33.3	
	Week 8	Peg	23	19 (82.6)	-1.75 (17.48)	-33.3	0.00	33.3	-0.38 (0.38) [-1.15, 0.34]
		SoC	16	13 (81.3)	5.13 (18.49)	-33.3	0.00	33.3	
	Week 12	Peg	23	20 (87.0)	3.33 (18.42)	-33.3	0.00	33.3	0.19 (0.37) [-0.52, 0.94]
		SoC	16	13 (81.3)	0.00 (13.61)	-33.3	0.00	33.3	
	Week 16	Peg	23	20 (87.0)	0.00 (18.73)	-33.3	0.00	33.3	-0.28 (0.36) [-1.02, 0.41]
		SoC	16	14 (87.5)	4.76 (12.10)	0.0	0.00	33.3	
	Week 20	Peg	23	22 (95.7)	3.03 (14.21)	-33.3	0.00	33.3	0.04 (0.36) [-0.67, 0.75]
		SoC	16	13 (81.3)	2.56 (9.25)	0.0	0.00	33.3	
	Week 26	Peg	23	18 (78.3)	-1.85 (17.98)	-33.3	0.00	33.3	-0.32 (0.41) [-1.16, 0.47]
		SoC	16	10 (62.5)	3.33 (10.54)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SICA: Change from baseline in QLQ-C30 Diarrhoea (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.2623

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SICA: Change from baseline in QLQ-C30 Diarrhoea (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 4	21	18 (85.7)	-6.32 (2.76)	(-12.08, -0.56)	-2.40 (5.95)	(-14.81, 10.01)	0.6913	
		Week 8	21	17 (81.0)	-3.31 (3.29)	(-10.19, 3.57)	-1.24 (6.91)	(-15.70, 13.22)	0.8596	
		Week 12	21	18 (85.7)	-4.56 (3.19)	(-11.22, 2.10)	-0.98 (6.87)	(-15.32, 13.36)	0.8880	
		Week 16	21	17 (81.0)	-5.17 (2.43)	(-10.24, -0.09)	-3.13 (4.78)	(-13.11, 6.84)	0.5199	
		Week 20	21	18 (85.7)	-1.62 (2.80)	(-7.46, 4.23)	-0.76 (6.03)	(-13.34, 11.82)	0.9006	
		Week 26	21	15 (71.4)	-10.82 (2.58)	(-16.31, -5.32)	-9.34 (6.32)	(-22.80, 4.12)	0.1600	
	SoC	Week 4	8	5 (62.5)	-3.92 (5.26)	(-14.89, 7.04)				
		Week 8	8	5 (62.5)	-2.07 (6.07)	(-14.78, 10.63)				
		Week 12	8	5 (62.5)	-3.58 (6.07)	(-16.25, 9.09)				
		Week 16	8	6 (75.0)	-2.03 (4.11)	(-10.60, 6.53)				
		Week 20	8	5 (62.5)	-0.85 (5.33)	(-11.97, 10.26)				
		Week 26	8	3 (37.5)	-1.48 (5.76)	(-13.77, 10.81)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SICA: Change from baseline in QLQ-C30 Diarrhoea (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	0.00 (2.66)	(-5.55, 5.55)	3.33 (4.17)	(-5.37, 12.04)	0.4337	
		Week 8	14	12 (85.7)	-1.52 (3.44)	(-8.71, 5.68)	-6.67 (5.29)	(-17.74, 4.41)	0.2228	
		Week 12	14	13 (92.9)	1.45 (2.69)	(-4.16, 7.06)	6.67 (4.22)	(-2.13, 15.46)	0.1295	
		Week 16	14	13 (92.9)	7.25 (5.36)	(-3.94, 18.43)	3.33 (8.41)	(-14.21, 20.88)	0.6961	
		Week 20	14	14 (100.0)	2.53 (3.56)	(-4.88, 9.93)	2.73 (5.69)	(-9.10, 14.56)	0.6366	
		Week 26	14	12 (85.7)	1.59 (3.65)	(-6.09, 9.26)	3.70 (5.79)	(-8.46, 15.87)	0.5305	
	SoC	Week 4	10	10 (100.0)	-3.33 (3.06)	(-9.71, 3.04)				
		Week 8	10	10 (100.0)	5.15 (3.79)	(-2.78, 13.08)				
		Week 12	10	10 (100.0)	-5.22 (3.09)	(-11.66, 1.23)				
		Week 16	10	10 (100.0)	3.91 (6.16)	(-8.94, 16.77)				
		Week 20	10	10 (100.0)	-0.20 (4.25)	(-9.04, 8.64)				
		Week 26	10	9 (90.0)	-2.12 (4.26)	(-11.06, 6.83)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SIHA: Change from baseline in QLQ-C30 Diarrhoea (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Diarrhoea (censored)	< 4	Baseline	Peg	21	21 (100.0)	9.52 (18.69)	0.0	0.00	66.7	
			SoC	8	6 (75.0)	5.56 (13.61)	0.0	0.00	33.3	
	Week 4		Peg	21	18 (85.7)	3.70 (10.78)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	4.76 (12.60)	0.0	0.00	33.3	
	Week 8		Peg	21	17 (81.0)	5.88 (13.10)	0.0	0.00	33.3	
			SoC	8	6 (75.0)	5.56 (13.61)	0.0	0.00	33.3	
	Week 12		Peg	21	18 (85.7)	5.56 (12.78)	0.0	0.00	33.3	
			SoC	8	6 (75.0)	5.56 (13.61)	0.0	0.00	33.3	
	Week 16		Peg	21	17 (81.0)	3.92 (11.07)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	4.76 (12.60)	0.0	0.00	33.3	
	Week 20		Peg	21	18 (85.7)	9.26 (19.15)	0.0	0.00	66.7	
			SoC	8	7 (87.5)	4.76 (12.60)	0.0	0.00	33.3	
	Week 26		Peg	21	15 (71.4)	2.22 (8.61)	0.0	0.00	33.3	
			SoC	8	4 (50.0)	8.33 (16.67)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SIHA: Change from baseline in QLQ-C30 Diarrhoea (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Diarrhoea (censored)	>= 4	Baseline	Peg	14	14 (100.0)	7.14 (14.19)	0.0	0.00	33.3	
			SoC	10	10 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 4	Peg	14	13 (92.9)	5.13 (12.52)	0.0	0.00	33.3		
		SoC	10	10 (100.0)	0.00 (0.00)	0.0	0.00	0.0		
	Week 8	Peg	14	12 (85.7)	5.56 (12.97)	0.0	0.00	33.3		
		SoC	10	10 (100.0)	6.67 (14.05)	0.0	0.00	33.3		
	Week 12	Peg	14	13 (92.9)	5.13 (12.52)	0.0	0.00	33.3		
		SoC	10	10 (100.0)	0.00 (0.00)	0.0	0.00	0.0		
	Week 16	Peg	14	13 (92.9)	12.82 (21.68)	0.0	0.00	66.7		
		SoC	10	10 (100.0)	6.67 (14.05)	0.0	0.00	33.3		
	Week 20	Peg	14	14 (100.0)	7.14 (14.19)	0.0	0.00	33.3		
		SoC	10	10 (100.0)	3.33 (10.54)	0.0	0.00	33.3		
	Week 26	Peg	14	12 (85.7)	5.56 (12.97)	0.0	0.00	33.3		
		SoC	10	9 (90.0)	3.70 (11.11)	0.0	0.00	33.3		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SIHA: Change from baseline in QLQ-C30 Diarrhoea (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in QLQ-C30 Diarrhoea (censored)	Week 4	Peg	21	18 (85.7)	-7.41 (24.40)	-66.7	0.00	33.3	-0.29 (0.53) [-1.38, 0.73]
		SoC	8	5 (62.5)	0.00 (23.57)	-33.3	0.00	33.3	
	Week 8	Peg	21	17 (81.0)	-3.92 (20.01)	-33.3	0.00	33.3	-0.18 (0.54) [-1.27, 0.86]
		SoC	8	5 (62.5)	0.00 (23.57)	-33.3	0.00	33.3	
	Week 12	Peg	21	18 (85.7)	-5.56 (23.57)	-66.7	0.00	33.3	-0.23 (0.53) [-1.31, 0.80]
		SoC	8	5 (62.5)	0.00 (23.57)	-33.3	0.00	33.3	
	Week 16	Peg	21	17 (81.0)	-5.88 (17.62)	-33.3	0.00	33.3	-0.37 (0.50) [-1.40, 0.59]
		SoC	8	6 (75.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 20	Peg	21	18 (85.7)	-1.85 (13.87)	-33.3	0.00	33.3	-0.14 (0.53) [-1.21, 0.89]
		SoC	8	5 (62.5)	0.00 (0.00)	0.0	0.00	0.0	
	Week 26	Peg	21	15 (71.4)	-11.11 (20.57)	-66.7	0.00	0.0	-0.55 (0.69) [-1.99, 0.73]
		SoC	8	3 (37.5)	0.00 (0.00)	0.0	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QHWC_SIHA: Change from baseline in QLQ-C30 Diarrhoea (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in QLQ-C30 Diarrhoea (censored)	Week 4	Peg	14	13 (92.9)	-2.56 (16.45)	-33.3	0.00	33.3	-0.20 (0.44) [-1.10, 0.66]
		SoC	10	10 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 8	Peg	14	12 (85.7)	-2.78 (9.62)	-33.3	0.00	0.0	-0.77 (0.47) [-1.79, 0.07]
		SoC	10	10 (100.0)	6.67 (14.05)	0.0	0.00	33.3	
	Week 12	Peg	14	13 (92.9)	-2.56 (21.35)	-33.3	0.00	33.3	-0.15 (0.44) [-1.05, 0.71]
		SoC	10	10 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 16	Peg	14	13 (92.9)	5.13 (22.96)	-33.3	0.00	33.3	-0.08 (0.44) [-0.96, 0.79]
		SoC	10	10 (100.0)	6.67 (14.05)	0.0	0.00	33.3	
	Week 20	Peg	14	14 (100.0)	0.00 (18.49)	-33.3	0.00	33.3	-0.20 (0.44) [-1.09, 0.63]
		SoC	10	10 (100.0)	3.33 (10.54)	0.0	0.00	33.3	
	Week 26	Peg	14	12 (85.7)	-2.78 (22.29)	-33.3	0.00	33.3	-0.34 (0.47) [-1.31, 0.55]
		SoC	10	9 (90.0)	3.70 (11.11)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SICC: Change from baseline in QLQ-C30 Financial Difficulties (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.1128

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SICC: Change from baseline in QLQ-C30 Financial Difficulties (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
< 60	Peg	Week 4	29	26 (89.7)	-19.00 (4.96)	(-29.10, -8.90)	-9.79 (9.27)	(-28.65, 9.08)	0.2990	
		Week 8	29	24 (82.8)	-30.39 (4.32)	(-39.20, -21.57)	-21.30 (7.84)	(-37.29, -5.32)	0.0106	*
		Week 12	29	26 (89.7)	-24.53 (3.83)	(-32.32, -16.74)	-16.14 (7.11)	(-30.61, -1.67)	0.0299	*
		Week 16	29	25 (86.2)	-25.00 (4.64)	(-34.43, -15.56)	-18.90 (8.18)	(-35.54, -2.27)	0.0272	*
		Week 20	29	27 (93.1)	-22.29 (4.47)	(-31.37, -13.20)	-10.32 (8.44)	(-27.46, 6.83)	0.2297	
	Week 26	29	23 (79.3)	-25.04 (4.92)	(-35.11, -14.97)	-8.83 (9.17)	(-27.58, 9.91)	0.3432		
	SoC	Week 4	13	11 (84.6)	-9.21 (7.69)	(-24.85, 6.43)				
		Week 8	13	11 (84.6)	-9.08 (6.42)	(-22.17, 4.00)				
		Week 12	13	11 (84.6)	-8.39 (5.91)	(-20.41, 3.63)				
		Week 16	13	12 (92.3)	-6.09 (6.69)	(-19.71, 7.52)				
		Week 20	13	11 (84.6)	-11.97 (7.07)	(-26.33, 2.40)				
Week 26	13	10 (76.9)	-16.20 (7.61)	(-31.77, -0.64)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SICC: Change from baseline in QLQ-C30 Financial Difficulties (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	0.34 (9.12)	(-23.10, 23.77)	3.03 (15.60)	(-37.08, 43.14)	0.8537	
		Week 8	6	5 (83.3)	5.00 (12.42)	(-26.93, 36.94)	2.16 (21.26)	(-52.50, 56.83)	0.9229	
		Week 12	6	5 (83.3)	-0.34 (9.12)	(-23.77, 23.10)	-3.03 (15.60)	(-43.14, 37.08)	0.8537	
		Week 16	6	5 (83.3)	20.14 (16.23)	(-21.58, 61.85)	19.86 (26.89)	(-49.26, 88.98)	0.4933	
		Week 20	6	5 (83.3)	-2.08 (13.98)	(-38.02, 33.85)	-4.58 (23.16)	(-64.13, 54.96)	0.8509	
		Week 26	6	4 (66.7)	16.67 (10.21)	(-27.25, 60.58)	50.00 (20.41)	(-37.83, 137.83)	0.1340	
	SoC	Week 4	5	4 (80.0)	-2.69 (10.65)	(-30.07, 24.68)				
		Week 8	5	4 (80.0)	2.84 (14.51)	(-34.47, 40.15)				
		Week 12	5	4 (80.0)	2.69 (10.65)	(-24.68, 30.07)				
		Week 16	5	4 (80.0)	0.28 (19.08)	(-48.76, 49.31)				
		Week 20	5	4 (80.0)	2.50 (16.43)	(-39.74, 44.74)				
		Week 26	5	2 (40.0)	-33.33 (15.59)	(-100.41, 33.75)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SIHC: Change from baseline in QLQ-C30 Financial Difficulties (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Financial Difficulties (censored)	< 60	Baseline	Peg	29	29 (100.0)	49.43 (32.88)	0.0	33.33	100.0	
			SoC	13	12 (92.3)	44.44 (38.49)	0.0	33.33	100.0	
		Week 4	Peg	29	26 (89.7)	28.21 (33.59)	0.0	33.33	100.0	
			SoC	13	12 (92.3)	33.33 (34.82)	0.0	33.33	100.0	
		Week 8	Peg	29	24 (82.8)	15.28 (25.97)	0.0	0.00	100.0	
			SoC	13	11 (84.6)	33.33 (36.51)	0.0	33.33	100.0	
		Week 12	Peg	29	26 (89.7)	19.23 (25.25)	0.0	0.00	100.0	
			SoC	13	11 (84.6)	33.33 (33.33)	0.0	33.33	100.0	
		Week 16	Peg	29	25 (86.2)	20.00 (27.22)	0.0	0.00	66.7	
			SoC	13	12 (92.3)	38.89 (39.78)	0.0	33.33	100.0	
		Week 20	Peg	29	27 (93.1)	23.46 (30.40)	0.0	0.00	100.0	
			SoC	13	12 (92.3)	30.56 (30.01)	0.0	33.33	100.0	
		Week 26	Peg	29	23 (79.3)	20.29 (26.09)	0.0	0.00	66.7	
			SoC	13	10 (76.9)	30.00 (33.15)	0.0	33.33	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SIHC: Change from baseline in QLQ-C30 Financial Difficulties (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Financial Difficulties (censored)	>= 60	Baseline	Peg	6	6 (100.0)	22.22 (27.22)	0.0	16.67	66.7	
			SoC	5	4 (80.0)	50.00 (33.33)	33.3	33.33	100.0	
		Week 4	Peg	6	5 (83.3)	13.33 (29.81)	0.0	0.00	66.7	
			SoC	5	5 (100.0)	40.00 (36.51)	0.0	33.33	100.0	
		Week 8	Peg	6	5 (83.3)	20.00 (18.26)	0.0	33.33	33.3	
			SoC	5	5 (100.0)	40.00 (43.46)	0.0	33.33	100.0	
		Week 12	Peg	6	5 (83.3)	13.33 (18.26)	0.0	0.00	33.3	
			SoC	5	5 (100.0)	40.00 (43.46)	0.0	33.33	100.0	
		Week 16	Peg	6	5 (83.3)	40.00 (43.46)	0.0	33.33	100.0	
			SoC	5	5 (100.0)	40.00 (43.46)	0.0	33.33	100.0	
		Week 20	Peg	6	5 (83.3)	20.00 (29.81)	0.0	0.00	66.7	
			SoC	5	5 (100.0)	40.00 (43.46)	0.0	33.33	100.0	
		Week 26	Peg	6	4 (66.7)	25.00 (31.91)	0.0	16.67	66.7	
			SoC	5	3 (60.0)	11.11 (19.25)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SIHC: Change from baseline in QLQ-C30 Financial Difficulties (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in QLQ-C30 Financial Difficulties (censored)	Week 4	Peg	29	26 (89.7)	-20.51 (29.93)	-100.0	0.00	33.3	-0.54 (0.38) [-1.31, 0.17]
		SoC	13	11 (84.6)	-6.06 (13.48)	-33.3	0.00	0.0	
	Week 8	Peg	29	24 (82.8)	-31.94 (26.88)	-100.0	-33.33	0.0	-1.07 (0.40) [-1.93, -0.35]
		SoC	13	11 (84.6)	-6.06 (13.48)	-33.3	0.00	0.0	
	Week 12	Peg	29	26 (89.7)	-25.64 (25.49)	-66.7	-33.33	33.3	-0.84 (0.39) [-1.66, -0.14]
		SoC	13	11 (84.6)	-6.06 (13.48)	-33.3	0.00	0.0	
	Week 16	Peg	29	25 (86.2)	-25.33 (29.31)	-100.0	-33.33	33.3	-0.76 (0.37) [-1.55, -0.08]
		SoC	13	12 (92.3)	-5.56 (12.97)	-33.3	0.00	0.0	
	Week 20	Peg	29	27 (93.1)	-23.46 (28.96)	-100.0	-33.33	33.3	-0.54 (0.37) [-1.31, 0.16]
		SoC	13	11 (84.6)	-9.09 (15.57)	-33.3	0.00	0.0	
	Week 26	Peg	29	23 (79.3)	-26.09 (30.08)	-100.0	-33.33	33.3	-0.44 (0.40) [-1.25, 0.31]
		SoC	13	10 (76.9)	-13.33 (23.31)	-66.7	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SIHC: Change from baseline in QLQ-C30 Financial Difficulties (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in QLQ-C30 Financial Difficulties (censored)	Week 4	Peg	6	5 (83.3)	0.00 (23.57)	-33.3	0.00	33.3	NE (NE) [NE, NE]
		SoC	5	4 (80.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 8	Peg	6	5 (83.3)	6.67 (14.91)	0.0	0.00	33.3	0.28 (0.80) [-1.21, 1.99]
		SoC	5	4 (80.0)	0.00 (27.22)	-33.3	0.00	33.3	
	Week 12	Peg	6	5 (83.3)	0.00 (0.00)	0.0	0.00	0.0	NE (NE) [NE, NE]
		SoC	5	4 (80.0)	0.00 (27.22)	-33.3	0.00	33.3	
	Week 16	Peg	6	5 (83.3)	20.00 (29.81)	0.0	0.00	66.7	0.62 (0.83) [-0.78, 2.50]
		SoC	5	4 (80.0)	0.00 (27.22)	-33.3	0.00	33.3	
	Week 20	Peg	6	5 (83.3)	0.00 (23.57)	-33.3	0.00	33.3	NE (NE) [NE, NE]
		SoC	5	4 (80.0)	0.00 (27.22)	-33.3	0.00	33.3	
	Week 26	Peg	6	4 (66.7)	16.67 (19.25)	0.0	16.67	33.3	1.31 (1.50) [-0.44, 5.15]
		SoC	5	2 (40.0)	-16.67 (23.57)	-33.3	-16.67	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SICD: Change from baseline in QLQ-C30 Financial Difficulties (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.9087

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SICD: Change from baseline in QLQ-C30 Financial Difficulties (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Male	Peg	Week 4	19	16 (84.2)	-15.07 (6.48)	(-28.59, -1.55)	-7.35 (11.19)	(-30.70, 16.00)	0.5188	
		Week 8	19	15 (78.9)	-16.74 (6.82)	(-31.02, -2.46)	-12.25 (11.61)	(-36.55, 12.05)	0.3045	
		Week 12	19	17 (89.5)	-15.40 (5.08)	(-25.96, -4.84)	-15.63 (9.00)	(-34.36, 3.10)	0.0972	
		Week 16	19	16 (84.2)	-21.24 (7.69)	(-37.24, -5.25)	-19.12 (12.99)	(-46.13, 7.89)	0.1559	
		Week 20	19	17 (89.5)	-18.78 (6.55)	(-32.40, -5.16)	-14.86 (11.62)	(-39.03, 9.30)	0.2148	
		Week 26	19	14 (73.7)	-17.75 (7.18)	(-32.96, -2.54)	-3.62 (13.13)	(-31.46, 24.22)	0.7864	
	SoC	Week 4	10	8 (80.0)	-7.72 (9.21)	(-26.94, 11.50)				
		Week 8	10	8 (80.0)	-4.49 (9.43)	(-24.23, 15.25)				
		Week 12	10	8 (80.0)	0.23 (7.47)	(-15.30, 15.77)				
		Week 16	10	9 (90.0)	-2.12 (10.46)	(-23.88, 19.64)				
		Week 20	10	8 (80.0)	-3.91 (9.64)	(-23.96, 16.13)				
		Week 26	10	6 (60.0)	-14.13 (10.98)	(-37.40, 9.14)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SICD: Change from baseline in QLQ-C30 Financial Difficulties (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Female	Peg	Week 4	16	15 (93.8)	-17.60 (5.65)	(-29.47, -5.73)	-16.39 (11.93)	(-41.46, 8.68)	0.1864	
		Week 8	16	14 (87.5)	-34.24 (4.77)	(-44.31, -24.16)	-31.34 (10.05)	(-52.54, -10.14)	0.0062	*
		Week 12	16	14 (87.5)	-29.33 (5.08)	(-40.05, -18.61)	-20.75 (10.69)	(-43.31, 1.80)	0.0690	
		Week 16	16	14 (87.5)	-14.80 (6.68)	(-28.89, -0.71)	-10.80 (14.05)	(-40.44, 18.84)	0.4527	
		Week 20	16	15 (93.8)	-20.39 (5.57)	(-32.09, -8.70)	-12.01 (11.76)	(-36.71, 12.70)	0.3208	
	Week 26	16	13 (81.3)	-22.09 (6.30)	(-35.52, -8.66)	-8.33 (13.38)	(-36.85, 20.20)	0.5431		
	SoC	Week 4	8	7 (87.5)	-1.21 (9.89)	(-21.98, 19.56)				
		Week 8	8	7 (87.5)	-2.90 (8.05)	(-19.88, 14.09)				
		Week 12	8	7 (87.5)	-8.57 (8.57)	(-26.64, 9.50)				
		Week 16	8	7 (87.5)	-4.00 (11.26)	(-27.75, 19.75)				
		Week 20	8	7 (87.5)	-8.39 (9.74)	(-28.85, 12.08)				
Week 26		8	6 (75.0)	-13.76 (11.00)	(-37.22, 9.69)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SIHD: Change from baseline in QLQ-C30 Financial Difficulties (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Financial Difficulties (censored)	Male	Baseline	Peg	19	19 (100.0)	42.11 (33.04)	0.0	33.33	100.0	
			SoC	10	9 (90.0)	44.44 (37.27)	0.0	33.33	100.0	
		Week 4	Peg	19	16 (84.2)	25.00 (31.03)	0.0	16.67	100.0	
			SoC	10	9 (90.0)	25.93 (32.39)	0.0	33.33	100.0	
		Week 8	Peg	19	15 (78.9)	24.44 (29.46)	0.0	33.33	100.0	
			SoC	10	9 (90.0)	29.63 (35.14)	0.0	33.33	100.0	
		Week 12	Peg	19	17 (89.5)	23.53 (28.30)	0.0	33.33	100.0	
			SoC	10	9 (90.0)	33.33 (37.27)	0.0	33.33	100.0	
		Week 16	Peg	19	16 (84.2)	20.83 (34.16)	0.0	0.00	100.0	
			SoC	10	10 (100.0)	36.67 (39.91)	0.0	33.33	100.0	
		Week 20	Peg	19	17 (89.5)	21.57 (31.05)	0.0	0.00	100.0	
			SoC	10	9 (90.0)	29.63 (35.14)	0.0	33.33	100.0	
		Week 26	Peg	19	14 (73.7)	21.43 (28.06)	0.0	0.00	66.7	
			SoC	10	7 (70.0)	23.81 (37.09)	0.0	0.00	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SIHD: Change from baseline in QLQ-C30 Financial Difficulties (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Financial Difficulties (censored)	Female	Baseline	Peg	16	16 (100.0)	47.92 (34.36)	0.0	33.33	100.0	
			SoC	8	7 (87.5)	47.62 (37.80)	0.0	33.33	100.0	
		Week 4	Peg	16	15 (93.8)	26.67 (36.08)	0.0	0.00	100.0	
			SoC	8	8 (100.0)	45.83 (35.36)	0.0	33.33	100.0	
		Week 8	Peg	16	14 (87.5)	7.14 (14.19)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	42.86 (41.79)	0.0	33.33	100.0	
		Week 12	Peg	16	14 (87.5)	11.90 (16.57)	0.0	0.00	33.3	
			SoC	8	7 (87.5)	38.10 (35.63)	0.0	33.33	100.0	
		Week 16	Peg	16	14 (87.5)	26.19 (26.73)	0.0	33.33	66.7	
			SoC	8	7 (87.5)	42.86 (41.79)	0.0	33.33	100.0	
		Week 20	Peg	16	15 (93.8)	24.44 (29.46)	0.0	0.00	66.7	
			SoC	8	8 (100.0)	37.50 (33.03)	0.0	33.33	100.0	
		Week 26	Peg	16	13 (81.3)	20.51 (25.60)	0.0	0.00	66.7	
			SoC	8	6 (75.0)	27.78 (25.09)	0.0	33.33	66.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SIHD: Change from baseline in QLQ-C30 Financial Difficulties (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Financial Difficulties (censored)	Male	Week 4	Peg	19	16 (84.2)	-16.67 (34.43)	-100.0	0.00	33.3	-0.27 (0.46) [-1.20, 0.60]
			SoC	10	8 (80.0)	-8.33 (15.43)	-33.3	0.00	0.0	
		Week 8	Peg	19	15 (78.9)	-17.78 (33.01)	-100.0	0.00	33.3	-0.44 (0.47) [-1.41, 0.43]
			SoC	10	8 (80.0)	-4.17 (21.36)	-33.3	0.00	33.3	
		Week 12	Peg	19	17 (89.5)	-15.69 (23.91)	-66.7	0.00	0.0	-0.68 (0.46) [-1.66, 0.16]
			SoC	10	8 (80.0)	0.00 (17.82)	-33.3	0.00	33.3	
		Week 16	Peg	19	16 (84.2)	-20.83 (38.25)	-100.0	-16.67	66.7	-0.50 (0.44) [-1.43, 0.32]
			SoC	10	9 (90.0)	-3.70 (20.03)	-33.3	0.00	33.3	
		Week 20	Peg	19	17 (89.5)	-19.61 (33.46)	-100.0	0.00	33.3	-0.49 (0.46) [-1.44, 0.36]
			SoC	10	8 (80.0)	-4.17 (21.36)	-33.3	0.00	33.3	
		Week 26	Peg	19	14 (73.7)	-16.67 (36.40)	-100.0	0.00	33.3	NE (NE) [NE, NE]
			SoC	10	6 (60.0)	-16.67 (27.89)	-66.7	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SIHD: Change from baseline in QLQ-C30 Financial Difficulties (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Financial Difficulties (censored)	Female	Week 4	Peg	16	15 (93.8)	-17.78 (24.77)	-66.7	-33.33	33.3	-0.83 (0.50) [-1.92, 0.07]
			SoC	8	7 (87.5)	0.00 (0.00)	0.0	0.00	0.0	
		Week 8	Peg	16	14 (87.5)	-33.33 (22.65)	-66.7	-33.33	0.0	-1.37 (0.55) [-2.62, -0.46]
			SoC	8	7 (87.5)	-4.76 (12.60)	-33.3	0.00	0.0	
		Week 12	Peg	16	14 (87.5)	-28.57 (25.68)	-66.7	-33.33	33.3	-0.79 (0.51) [-1.90, 0.12]
			SoC	8	7 (87.5)	-9.52 (16.27)	-33.3	0.00	0.0	
		Week 16	Peg	16	14 (87.5)	-14.29 (28.39)	-66.7	-16.67	33.3	-0.37 (0.49) [-1.40, 0.56]
			SoC	8	7 (87.5)	-4.76 (12.60)	-33.3	0.00	0.0	
		Week 20	Peg	16	15 (93.8)	-20.00 (24.56)	-66.7	-33.33	33.3	-0.45 (0.49) [-1.47, 0.46]
			SoC	8	7 (87.5)	-9.52 (16.27)	-33.3	0.00	0.0	
		Week 26	Peg	16	13 (81.3)	-23.08 (28.50)	-66.7	-33.33	33.3	-0.44 (0.53) [-1.56, 0.55]
			SoC	8	6 (75.0)	-11.11 (17.21)	-33.3	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SICB: Change from baseline in QLQ-C30 Financial Difficulties (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.0702

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SICB: Change from baseline in QLQ-C30 Financial Difficulties (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Latin America	Peg	Week 4	12	11 (91.7)	-23.55 (7.28)	(-40.02, -7.08)	-26.26 (18.52)	(-68.15, 15.63)	0.1899	
		Week 8	12	10 (83.3)	-37.04 (7.50)	(-54.32, -19.75)	-38.88 (18.38)	(-81.26, 3.50)	0.0673	
		Week 12	12	11 (91.7)	-27.44 (5.92)	(-40.82, -14.06)	-29.21 (15.05)	(-63.25, 4.83)	0.0841	
		Week 16	12	10 (83.3)	-33.70 (9.80)	(-56.31, -11.09)	-35.54 (24.03)	(-90.95, 19.87)	0.1774	
		Week 20	12	10 (83.3)	-30.79 (9.13)	(-51.85, -9.72)	-34.72 (22.44)	(-86.46, 17.02)	0.1603	
		Week 26	12	9 (75.0)	-29.18 (9.42)	(-51.46, -6.89)	-32.18 (22.02)	(-84.24, 19.89)	0.1873	
	SoC	Week 4	2	2 (100.0)	2.71 (17.03)	(-35.82, 41.24)				
		Week 8	2	2 (100.0)	1.84 (16.77)	(-36.83, 40.52)				
		Week 12	2	2 (100.0)	1.77 (13.84)	(-29.54, 33.07)				
		Week 16	2	2 (100.0)	1.84 (21.93)	(-48.74, 52.42)				
		Week 20	2	2 (100.0)	3.94 (20.47)	(-43.27, 51.14)				
		Week 26	2	2 (100.0)	3.00 (19.92)	(-44.11, 50.10)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SICB: Change from baseline in QLQ-C30 Financial Difficulties (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Asia Pacific Peg		Week 4	23	20 (87.0)	-12.16 (5.04)	(-22.48, -1.85)	-4.80 (8.28)	(-21.72, 12.13)	0.5667	
		Week 8	23	19 (82.6)	-18.29 (5.00)	(-28.54, -8.04)	-11.68 (8.11)	(-28.28, 4.93)	0.1608	
		Week 12	23	20 (87.0)	-18.38 (4.72)	(-28.03, -8.72)	-13.11 (7.78)	(-29.01, 2.79)	0.1024	
		Week 16	23	20 (87.0)	-9.76 (5.49)	(-20.98, 1.46)	-4.66 (8.77)	(-22.56, 13.25)	0.5992	
		Week 20	23	22 (95.7)	-14.35 (4.58)	(-23.69, -5.02)	-5.04 (7.72)	(-20.79, 10.72)	0.5190	
		Week 26	23	18 (78.3)	-14.21 (5.27)	(-25.10, -3.33)	6.40 (9.44)	(-13.08, 25.89)	0.5041	
SoC		Week 4	16	13 (81.3)	-7.37 (6.37)	(-20.39, 5.66)				
		Week 8	16	13 (81.3)	-6.61 (6.11)	(-19.13, 5.91)				
		Week 12	16	13 (81.3)	-5.26 (5.97)	(-17.47, 6.95)				
		Week 16	16	14 (87.5)	-5.10 (6.61)	(-18.61, 8.40)				
		Week 20	16	13 (81.3)	-9.32 (6.06)	(-21.68, 3.05)				
		Week 26	16	10 (62.5)	-20.62 (7.62)	(-36.35, -4.89)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SIHB: Change from baseline in QLQ-C30 Financial Difficulties (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Financial Difficulties (censored)	Latin America	Baseline	Peg	12	12 (100.0)	50.00 (33.33)	0.0	50.00	100.0	
			SoC	2	2 (100.0)	50.00 (70.71)	0.0	50.00	100.0	
		Week 4	Peg	12	11 (91.7)	21.21 (22.47)	0.0	33.33	66.7	
			SoC	2	2 (100.0)	50.00 (70.71)	0.0	50.00	100.0	
		Week 8	Peg	12	10 (83.3)	10.00 (16.10)	0.0	0.00	33.3	
			SoC	2	2 (100.0)	50.00 (70.71)	0.0	50.00	100.0	
		Week 12	Peg	12	11 (91.7)	18.18 (17.41)	0.0	33.33	33.3	
			SoC	2	2 (100.0)	50.00 (70.71)	0.0	50.00	100.0	
		Week 16	Peg	12	10 (83.3)	13.33 (23.31)	0.0	0.00	66.7	
			SoC	2	2 (100.0)	50.00 (70.71)	0.0	50.00	100.0	
		Week 20	Peg	12	10 (83.3)	13.33 (23.31)	0.0	0.00	66.7	
			SoC	2	2 (100.0)	50.00 (70.71)	0.0	50.00	100.0	
		Week 26	Peg	12	9 (75.0)	14.81 (24.22)	0.0	0.00	66.7	
			SoC	2	2 (100.0)	50.00 (70.71)	0.0	50.00	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SIHB: Change from baseline in QLQ-C30 Financial Difficulties (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Financial Difficulties (censored)	Asia	Baseline	Peg	23	23 (100.0)	42.03 (33.66)	0.0	33.33	100.0	
			SoC	16	14 (87.5)	45.24 (33.61)	0.0	33.33	100.0	
	Pacific	Week 4	Peg	23	20 (87.0)	28.33 (37.89)	0.0	0.00	100.0	
			SoC	16	15 (93.8)	33.33 (30.86)	0.0	33.33	100.0	
		Week 8	Peg	23	19 (82.6)	19.30 (27.92)	0.0	0.00	100.0	
			SoC	16	14 (87.5)	33.33 (34.59)	0.0	33.33	100.0	
		Week 12	Peg	23	20 (87.0)	18.33 (27.52)	0.0	0.00	100.0	
			SoC	16	14 (87.5)	33.33 (32.03)	0.0	33.33	100.0	
		Week 16	Peg	23	20 (87.0)	28.33 (32.94)	0.0	16.67	100.0	
			SoC	16	15 (93.8)	37.78 (37.52)	0.0	33.33	100.0	
		Week 20	Peg	23	22 (95.7)	27.27 (31.93)	0.0	16.67	100.0	
			SoC	16	15 (93.8)	31.11 (29.46)	0.0	33.33	100.0	
		Week 26	Peg	23	18 (78.3)	24.07 (27.55)	0.0	16.67	66.7	
			SoC	16	11 (68.8)	21.21 (22.47)	0.0	33.33	66.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SIHB: Change from baseline in QLQ-C30 Financial Difficulties (censored) by region
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Latin in QLQ-C30 Financial America Difficulties (censored)	Week 4	Peg	12	11 (91.7)	-24.24 (33.63)	-100.0	0.00	0.0	-0.70 (0.87) [-2.59, 0.86]
		SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 8	Peg	12	10 (83.3)	-36.67 (33.15)	-100.0	-33.33	0.0	-1.08 (0.92) [-3.16, 0.47]
		SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 12	Peg	12	11 (91.7)	-27.27 (25.03)	-66.7	-33.33	0.0	-1.06 (0.89) [-3.07, 0.47]
		SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 16	Peg	12	10 (83.3)	-33.33 (38.49)	-100.0	-33.33	33.3	-0.84 (0.90) [-2.84, 0.72]
		SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 20	Peg	12	10 (83.3)	-30.00 (36.68)	-100.0	-16.67	0.0	-0.80 (0.89) [-2.77, 0.78]
		SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	
	Week 26	Peg	12	9 (75.0)	-29.63 (35.14)	-100.0	-33.33	0.0	-0.82 (0.92) [-2.88, 0.77]
		SoC	2	2 (100.0)	0.00 (0.00)	0.0	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SIHB: Change from baseline in QLQ-C30 Financial Difficulties (censored) by region
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Asia in QLQ-C30 Financial Pacific Difficulties (censored)	Week 4	Peg	23	20 (87.0)	-13.33 (27.36)	-66.7	0.00	33.3	-0.35 (0.37) [-1.11, 0.36]
		SoC	16	13 (81.3)	-5.13 (12.52)	-33.3	0.00	0.0	
	Week 8	Peg	23	19 (82.6)	-19.30 (25.62)	-66.7	-33.33	33.3	-0.60 (0.38) [-1.40, 0.11]
		SoC	16	13 (81.3)	-5.13 (18.49)	-33.3	0.00	33.3	
	Week 12	Peg	23	20 (87.0)	-18.33 (25.31)	-66.7	-16.67	33.3	-0.56 (0.38) [-1.34, 0.14]
		SoC	16	13 (81.3)	-5.13 (18.49)	-33.3	0.00	33.3	
	Week 16	Peg	23	20 (87.0)	-10.00 (28.82)	-66.7	0.00	66.7	-0.21 (0.36) [-0.93, 0.49]
		SoC	16	14 (87.5)	-4.76 (17.82)	-33.3	0.00	33.3	
	Week 20	Peg	23	22 (95.7)	-15.15 (24.62)	-66.7	-16.67	33.3	-0.32 (0.36) [-1.05, 0.38]
		SoC	16	13 (81.3)	-7.69 (19.97)	-33.3	0.00	33.3	
	Week 26	Peg	23	18 (78.3)	-14.81 (30.73)	-66.7	-16.67	33.3	0.06 (0.41) [-0.74, 0.88]
		SoC	16	10 (62.5)	-16.67 (23.57)	-66.7	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SICA: Change from baseline in QLQ-C30 Financial Difficulties (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.7431

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SICA: Change from baseline in QLQ-C30 Financial Difficulties (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 4	21	18 (85.7)	-20.18 (5.69)	(-32.05, -8.31)	-6.16 (12.43)	(-32.09, 19.78)	0.6260	
		Week 8	21	17 (81.0)	-27.05 (4.73)	(-36.95, -17.14)	-19.00 (10.12)	(-40.19, 2.19)	0.0760	
		Week 12	21	18 (85.7)	-24.25 (4.18)	(-32.97, -15.53)	-24.88 (9.13)	(-43.93, -5.84)	0.0130	*
		Week 16	21	17 (81.0)	-20.85 (6.10)	(-33.58, -8.12)	-18.80 (11.98)	(-43.79, 6.18)	0.1322	
		Week 20	21	18 (85.7)	-22.09 (5.20)	(-32.93, -11.25)	-14.93 (11.40)	(-38.71, 8.85)	0.2050	
	Week 26	21	15 (71.4)	-25.34 (5.96)	(-38.05, -12.63)	3.53 (14.71)	(-27.83, 34.89)	0.8138		
	SoC	Week 4	8	5 (62.5)	-14.02 (10.94)	(-36.85, 8.81)				
		Week 8	8	5 (62.5)	-8.05 (8.85)	(-26.57, 10.47)				
		Week 12	8	5 (62.5)	0.63 (8.04)	(-16.13, 17.39)				
		Week 16	8	6 (75.0)	-2.04 (10.29)	(-23.51, 19.42)				
		Week 20	8	5 (62.5)	-7.15 (10.02)	(-28.06, 13.75)				
Week 26	8	3 (37.5)	-28.86 (13.41)	(-57.45, -0.28)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SICA: Change from baseline in QLQ-C30 Financial Difficulties (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	-10.41 (5.71)	(-22.32, 1.50)	-7.27 (8.68)	(-25.38, 10.84)	0.4121	
		Week 8	14	12 (85.7)	-20.40 (6.95)	(-34.96, -5.85)	-14.89 (10.40)	(-36.65, 6.87)	0.1683	
		Week 12	14	13 (92.9)	-16.83 (5.96)	(-29.26, -4.39)	-8.70 (9.16)	(-27.80, 10.40)	0.3533	
		Week 16	14	13 (92.9)	-14.43 (8.14)	(-31.41, 2.56)	-9.85 (12.51)	(-35.95, 16.25)	0.4403	
		Week 20	14	14 (100.0)	-15.28 (6.75)	(-29.32, -1.23)	-6.67 (10.52)	(-28.55, 15.21)	0.5332	
	Week 26	14	12 (85.7)	-12.56 (7.04)	(-27.34, 2.23)	-3.38 (10.80)	(-26.07, 19.32)	0.7581		
	SoC	Week 4	10	10 (100.0)	-3.14 (6.51)	(-16.72, 10.45)				
		Week 8	10	10 (100.0)	-5.51 (7.63)	(-21.48, 10.45)				
		Week 12	10	10 (100.0)	-8.13 (6.82)	(-22.34, 6.09)				
		Week 16	10	10 (100.0)	-4.58 (9.31)	(-24.00, 14.85)				
		Week 20	10	10 (100.0)	-8.61 (8.00)	(-25.26, 8.03)				
	Week 26	10	9 (90.0)	-9.18 (8.14)	(-26.28, 7.91)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SIHA: Change from baseline in QLQ-C30 Financial Difficulties (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Financial Difficulties (censored)	< 4	Baseline	Peg	21	21 (100.0)	47.62 (32.61)	0.0	33.33	100.0	
			SoC	8	6 (75.0)	38.89 (38.97)	0.0	33.33	100.0	
		Week 4	Peg	21	18 (85.7)	20.37 (28.33)	0.0	0.00	100.0	
			SoC	8	7 (87.5)	19.05 (17.82)	0.0	33.33	33.3	
		Week 8	Peg	21	17 (81.0)	13.73 (20.61)	0.0	0.00	66.7	
			SoC	8	6 (75.0)	22.22 (27.22)	0.0	16.67	66.7	
		Week 12	Peg	21	18 (85.7)	16.67 (20.61)	0.0	0.00	66.7	
			SoC	8	6 (75.0)	27.78 (32.77)	0.0	16.67	66.7	
		Week 16	Peg	21	17 (81.0)	23.53 (28.30)	0.0	0.00	66.7	
			SoC	8	7 (87.5)	33.33 (38.49)	0.0	33.33	100.0	
		Week 20	Peg	21	18 (85.7)	20.37 (25.92)	0.0	0.00	66.7	
			SoC	8	7 (87.5)	23.81 (25.20)	0.0	33.33	66.7	
		Week 26	Peg	21	15 (71.4)	17.78 (24.77)	0.0	0.00	66.7	
			SoC	8	4 (50.0)	8.33 (16.67)	0.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SIHA: Change from baseline in QLQ-C30 Financial Difficulties (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Financial Difficulties (censored)	>= 4	Baseline	Peg	14	14 (100.0)	40.48 (35.03)	0.0	33.33	100.0	
			SoC	10	10 (100.0)	50.00 (36.00)	0.0	33.33	100.0	
	Week 4	Peg	14	13 (92.9)	33.33 (38.49)	0.0	33.33	100.0		
		SoC	10	10 (100.0)	46.67 (39.13)	0.0	33.33	100.0		
	Week 8	Peg	14	12 (85.7)	19.44 (30.01)	0.0	0.00	100.0		
		SoC	10	10 (100.0)	43.33 (41.72)	0.0	33.33	100.0		
	Week 12	Peg	14	13 (92.9)	20.51 (28.99)	0.0	0.00	100.0		
		SoC	10	10 (100.0)	40.00 (37.84)	0.0	33.33	100.0		
	Week 16	Peg	14	13 (92.9)	23.08 (34.39)	0.0	0.00	100.0		
		SoC	10	10 (100.0)	43.33 (41.72)	0.0	33.33	100.0		
	Week 20	Peg	14	14 (100.0)	26.19 (35.03)	0.0	0.00	100.0		
		SoC	10	10 (100.0)	40.00 (37.84)	0.0	33.33	100.0		
	Week 26	Peg	14	12 (85.7)	25.00 (28.87)	0.0	16.67	66.7		
		SoC	10	9 (90.0)	33.33 (33.33)	0.0	33.33	100.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SIHA: Change from baseline in QLQ-C30 Financial Difficulties (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in QLQ-C30 Financial Difficulties (censored)	Week 4	Peg	21	18 (85.7)	-22.22 (32.34)	-100.0	-16.67	33.3	-0.50 (0.54) [-1.62, 0.51]
		SoC	8	5 (62.5)	-6.67 (14.91)	-33.3	0.00	0.0	
	Week 8	Peg	21	17 (81.0)	-29.41 (28.58)	-100.0	-33.33	0.0	-1.02 (0.57) [-2.26, -0.03]
		SoC	8	5 (62.5)	0.00 (23.57)	-33.3	0.00	33.3	
	Week 12	Peg	21	18 (85.7)	-25.93 (24.40)	-66.7	-33.33	0.0	-1.37 (0.58) [-2.67, -0.38]
		SoC	8	5 (62.5)	6.67 (14.91)	0.0	0.00	33.3	
	Week 16	Peg	21	17 (81.0)	-21.57 (31.05)	-100.0	-33.33	33.3	-0.72 (0.51) [-1.81, 0.22]
		SoC	8	6 (75.0)	0.00 (21.08)	-33.3	0.00	33.3	
	Week 20	Peg	21	18 (85.7)	-24.07 (27.55)	-100.0	-33.33	0.0	-0.86 (0.55) [-2.05, 0.13]
		SoC	8	5 (62.5)	0.00 (23.57)	-33.3	0.00	33.3	
	Week 26	Peg	21	15 (71.4)	-26.67 (31.37)	-100.0	-33.33	0.0	-0.13 (0.68) [-1.49, 1.19]
		SoC	8	3 (37.5)	-22.22 (38.49)	-66.7	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QMWC_SIHA: Change from baseline in QLQ-C30 Financial Difficulties (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline \geq 4 in QLQ-C30 Financial Difficulties (censored)	Week 4	Peg	14	13 (92.9)	-10.26 (25.04)	-66.7	0.00	33.3	-0.33 (0.45) [-1.25, 0.51]
		SoC	10	10 (100.0)	-3.33 (10.54)	-33.3	0.00	0.0	
	Week 8	Peg	14	12 (85.7)	-19.44 (30.01)	-66.7	-16.67	33.3	-0.51 (0.46) [-1.48, 0.34]
		SoC	10	10 (100.0)	-6.67 (14.05)	-33.3	0.00	0.0	
	Week 12	Peg	14	13 (92.9)	-15.38 (25.88)	-66.7	0.00	33.3	-0.23 (0.44) [-1.14, 0.62]
		SoC	10	10 (100.0)	-10.00 (16.10)	-33.3	0.00	0.0	
	Week 16	Peg	14	13 (92.9)	-12.82 (37.36)	-66.7	0.00	66.7	-0.20 (0.44) [-1.10, 0.66]
		SoC	10	10 (100.0)	-6.67 (14.05)	-33.3	0.00	0.0	
	Week 20	Peg	14	14 (100.0)	-14.29 (31.25)	-66.7	0.00	33.3	-0.16 (0.44) [-1.04, 0.69]
		SoC	10	10 (100.0)	-10.00 (16.10)	-33.3	0.00	0.0	
	Week 26	Peg	14	12 (85.7)	-11.11 (32.82)	-66.7	-16.67	33.3	0.00 (0.47) [-0.92, 0.92]
		SoC	10	9 (90.0)	-11.11 (16.67)	-33.3	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QPWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								0.0764

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	10 (34.5)	4.43	(4.14, 12.14)	1.21	(0.40, 3.62)	0.7365	
	SoC	13	5 (38.5)	4.29	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	1 (16.7)	NE	(4.57, NE)	0.16	(0.01, 4.11)	0.2710	
	SoC	5	3 (60.0)	4.14	(3.86, 8.57)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by sex
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.5502

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by sex
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	4 (21.1)	4.57	(4.14, NE)	0.53	(0.14, 2.08)	0.3636	
	SoC	10	5 (50.0)	4.21	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by sex
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	7 (43.8)	4.57	(4.14, 12.14)	0.86	(0.17, 4.31)	0.8538	
	SoC	8	3 (37.5)	6.36	(3.86, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								0.8329

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	5 (41.7)	4.29	(4.14, 4.57)	0.27	(0.02, 3.32)	0.3032	
	SoC	2	1 (50.0)	4.14	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	6 (26.1)	12.14	(4.14, NE)	0.70	(0.22, 2.20)	0.5448	
	SoC	16	7 (43.8)	4.29	(3.86, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWDT10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								0.2009

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWDT10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	7 (33.3)	4.57	(4.14, NE)	0.49	(0.15, 1.57)	0.2302	
	SoC	8	5 (62.5)	4.14	(4.14, 16.43)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWDT10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	4 (28.6)	8.21	(4.14, 12.14)	1.62	(0.36, 7.31)	0.5331	
	SoC	10	3 (30.0)	8.57	(3.86, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								0.6168

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	25 (86.2)	4.14	(4.14, 4.29)	2.36	(1.00, 5.55)	0.0497	*
	SoC	13	7 (53.8)	12.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	2 (33.3)	NE	(4.29, NE)	1.93	(0.16, 23.73)	0.6091	
	SoC	5	1 (20.0)	NE	(3.86, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by sex
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.4793

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by sex
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	12 (63.2)	4.57	(4.14, NE)	1.74	(0.56, 5.43)	0.3387	
	SoC	10	4 (40.0)	12.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by sex
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	15 (93.8)	4.14	(NE, NE)	3.40	(0.91, 12.73)	0.0690	
	SoC	8	4 (50.0)	16.14	(3.86, 16.14)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								0.1726

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	10 (83.3)	4.21	(4.14, 4.57)	0.29	(0.03, 2.85)	0.2913	
	SoC	2	1 (50.0)	4.14	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	17 (73.9)	4.14	(4.14, 12.14)	2.00	(0.81, 4.93)	0.1324	
	SoC	16	7 (43.8)	16.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWDT10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								0.8993

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWDT10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	16 (76.2)	4.14	(4.14, 8.14)	2.23	(0.64, 7.71)	0.2055	
	SoC	8	3 (37.5)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWDT10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	11 (78.6)	4.14	(4.14, 4.29)	1.96	(0.67, 5.69)	0.2183	
	SoC	10	5 (50.0)	12.14	(3.86, 16.14)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QDWD10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Dyspnoea (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Dyspnoea (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QDWD10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Dyspnoea (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Dyspnoea (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	10 (34.5)	4.14	(4.14, 4.57)	4.62	(1.23, 17.41)	0.0238	*
	SoC	13	3 (23.1)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QDWD10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Dyspnoea (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Dyspnoea (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	2 (33.3)	12.14	(4.57, NE)	NE	(NE, NE)	NE	
	SoC	5	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	13 (44.8)	4.14	(4.14, 11.57)	2.38	(0.77, 7.35)	0.1329	
	SoC	13	4 (30.8)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	1 (16.7)	12.14	(NE, NE)	NE	(NE, NE)	NE	
	SoC	5	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by sex
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.3492

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by sex
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	6 (31.6)	4.14	(4.14, 11.57)	5.12	(1.00, 26.24)	0.0501	
	SoC	10	2 (20.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by sex
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	8 (50.0)	8.14	(3.86, 12.14)	1.50	(0.21, 10.85)	0.6897	
	SoC	8	2 (25.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	5 (41.7)	4.14	(4.14, 12.14)	NE	(NE, NE)	NE	
	SoC	2	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	9 (39.1)	8.14	(4.14, 12.14)	3.11	(0.93, 10.42)	0.0654	
	SoC	16	4 (25.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWDT10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								0.8716

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWDT10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	9 (42.9)	6.14	(4.14, 12.14)	3.28	(0.70, 15.38)	0.1320	
	SoC	8	2 (25.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWDT10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	5 (35.7)	7.86	(3.86, NE)	3.30	(0.63, 17.29)	0.1583	
	SoC	10	2 (20.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QAWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QAWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored) by age ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	8 (27.6)	4.14	(3.86, 20.14)	2.30	(0.54, 9.82)	0.2598	
	SoC	13	3 (23.1)	12.29	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QAWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored) by age ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	1 (16.7)	4.57	(NE, NE)	NE	(NE, NE)	NE	
	SoC	5	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QCWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Constipation (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Constipation (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QCWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Constipation (censored) by age ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Constipation (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	9 (31.0)	4.14	(4.00, 4.29)	2.80	(0.34, 23.11)	0.3398	
	SoC	13	2 (15.4)	8.14	(4.14, 12.14)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QCWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Constipation (censored) by age ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Constipation (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	0 (0.0)	NE	(NE, NE)	NE	(NE, NE)	NE	
	SoC	5	2 (40.0)	8.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QHWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Diarrhoea (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Diarrhoea (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QHWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Diarrhoea (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Diarrhoea (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	8 (27.6)	4.14	(4.14, 4.57)	1.76	(0.20, 15.92)	0.6140	
	SoC	13	1 (7.7)	4.29	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QHWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Diarrhoea (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Diarrhoea (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	0 (0.0)	NE	(NE, NE)	NE	(NE, NE)	NE	
	SoC	5	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QMWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)
 by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								0.9667

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QMWD10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)
 by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	21 (72.4)	4.29	(4.14, 8.14)	2.41	(0.82, 7.06)	0.1086	
	SoC	13	4 (30.8)	12.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QMWD10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)
 by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	2 (33.3)	20.14	(4.00, NE)	2.10	(0.18, 24.66)	0.5535	
	SoC	5	1 (20.0)	NE	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QMWD10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)
 by sex
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.2942

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QMWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)
 by sex
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	11 (57.9)	12.14	(4.14, 20.43)	1.69	(0.46, 6.18)	0.4284	
	SoC	10	3 (30.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QMWD10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)
 by sex
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	12 (75.0)	4.14	(4.00, 8.14)	3.26	(0.59, 18.02)	0.1760	
	SoC	8	2 (25.0)	12.14	(8.14, 12.14)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QMWD10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)
 by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QMWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)
 by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	8 (66.7)	4.29	(4.14, 16.14)	NE	(NE, NE)	NE	
	SoC	2	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.

95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).

A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).

Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.

No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.

Source Data: apro_tte, created on: 22SEP2023

Table PT1QMWD10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)
 by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	15 (65.2)	8.14	(4.14, 19.86)	2.36	(0.84, 6.62)	0.1022	
	SoC	16	5 (31.3)	12.14	(4.29, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QMWD10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								0.2450

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QMWD10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	14 (66.7)	4.14	(4.14, 8.29)	5.65	(0.74, 43.18)	0.0950	
	SoC	8	1 (12.5)	NE	(4.29, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QMWD10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Financial Difficulties (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	9 (64.3)	8.14	(4.14, 16.14)	1.23	(0.34, 4.45)	0.7494	
	SoC	10	4 (40.0)	12.14	(4.14, 12.14)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by age
 ITT

Time to increase of at least 10 points in
 change from baseline of QLQ-C30 Pain
 (censored)

Subgroup	Treatment	N	nev (%)	Kaplan-Meier analysis		Cox model				
				Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA	
Age	Peg									0.7461

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by age
 ITT

Time to increase of at least 10 points in
 change from baseline of QLQ-C30 Pain
 (censored)

Subgroup	Treatment	N	nev (%)	Kaplan-Meier analysis		Cox model			
				Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	16 (55.2)	18.21	(8.14, NE)	1.45	(0.48, 4.37)	0.5057	
	SoC	13	4 (30.8)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.

95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).

A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).

Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.

No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.

Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by age
 ITT

Time to increase of at least 10 points in
 change from baseline of QLQ-C30 Pain
 (censored)

Subgroup	Treatment	N	nev (%)	Kaplan-Meier analysis		Cox model			
				Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	3 (50.0)	20.14	(4.29, NE)	0.31	(0.02, 5.06)	0.4144	
	SoC	5	2 (40.0)	12.14	(8.29, 12.14)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.

95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).

A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).

Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.

No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.

Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.3658

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	14 (73.7)	12.14	(8.14, 26.14)	1.75	(0.48, 6.37)	0.3976	
	SoC	10	3 (30.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	5 (31.3)	NE	(4.14, NE)	1.12	(0.22, 5.67)	0.8888	
	SoC	8	3 (37.5)	8.29	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								0.1916

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	6 (50.0)	26.14	(8.14, NE)	0.15	(0.01, 2.51)	0.1893	
	SoC	2	1 (50.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	13 (56.5)	20.14	(4.29, NE)	1.50	(0.53, 4.27)	0.4465	
	SoC	16	5 (31.3)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								0.2581

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	10 (47.6)	26.14	(8.29, NE)	2.98	(0.38, 23.34)	0.2975	
	SoC	8	1 (12.5)	NE	(12.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QPWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Pain (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	9 (64.3)	12.14	(4.14, NE)	0.92	(0.30, 2.84)	0.8844	
	SoC	10	5 (50.0)	8.29	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								0.5393

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	6 (20.7)	NE	(NE, NE)	0.33	(0.10, 1.03)	0.0571	
	SoC	13	6 (46.2)	12.29	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	2 (33.3)	NE	(4.14, NE)	0.17	(0.01, 2.18)	0.1754	
	SoC	5	3 (60.0)	4.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	5 (26.3)	NE	(12.29, NE)	0.24	(0.07, 0.83)	0.0249	*
	SoC	10	6 (60.0)	8.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	3 (18.8)	NE	(12.14, NE)	NE	(NE, NE)	NE	
	SoC	8	3 (37.5)	8.29	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								0.2435

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	1 (8.3)	NE	(NE, NE)	0.16	(0.01, 2.57)	0.1957	
	SoC	2	1 (50.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QFWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Fatigue (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	7 (30.4)	NE	(12.29, NE)	0.38	(0.14, 1.09)	0.0720	
	SoC	16	8 (50.0)	8.29	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QVWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Nausea/Vomiting (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Nausea/Vomiting (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								0.8153

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QVWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Nausea/Vomiting (censored) by age ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Nausea/Vomiting (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	7 (24.1)	NE	(NE, NE)	0.56	(0.14, 2.25)	0.4175	
	SoC	13	3 (23.1)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QVWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Nausea/Vomiting (censored) by age ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Nausea/Vomiting (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	3 (50.0)	20.14	(4.14, NE)	1.25	(0.14, 10.90)	0.8398	
	SoC	5	2 (40.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QVWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Nausea/Vomiting (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Nausea/Vomiting (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.2869

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QVWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Nausea/Vomiting (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Nausea/Vomiting (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	8 (42.1)	NE	(20.14, NE)	0.82	(0.21, 3.13)	0.7699	
	SoC	10	3 (30.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QVWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Nausea/Vomiting (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Nausea/Vomiting (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	2 (12.5)	NE	(NE, NE)	0.63	(0.08, 4.80)	0.6541	
	SoC	8	2 (25.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QVWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Nausea/Vomiting (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Nausea/Vomiting (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QVWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Nausea/Vomiting (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Nausea/Vomiting (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	1 (8.3)	NE	(NE, NE)	NE	(NE, NE)	NE	
	SoC	2	1 (50.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QVWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Nausea/Vomiting (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Nausea/Vomiting (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	9 (39.1)	NE	(20.14, NE)	0.89	(0.26, 3.04)	0.8558	
	SoC	16	4 (25.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QDWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Dyspnoea (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Dyspnoea (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QDWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Dyspnoea (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Dyspnoea (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	7 (24.1)	NE	(NE, NE)	0.36	(0.11, 1.15)	0.0846	
	SoC	13	5 (38.5)	16.00	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QDWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Dyspnoea (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Dyspnoea (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	0 (0.0)	NE	(NE, NE)	NE	(NE, NE)	NE	
	SoC	5	2 (40.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QDWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Dyspnoea (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Dyspnoea (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QDWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Dyspnoea (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Dyspnoea (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	0 (0.0)	NE	(NE, NE)	NE	(NE, NE)	NE	
	SoC	2	1 (50.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QDWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Dyspnoea (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Dyspnoea (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	7 (30.4)	NE	(20.43, NE)	0.42	(0.14, 1.28)	0.1265	
	SoC	16	6 (37.5)	16.00	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								0.4406

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	8 (27.6)	NE	(26.14, NE)	0.56	(0.17, 1.92)	0.3596	
	SoC	13	4 (30.8)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	4 (66.7)	12.36	(4.14, NE)	3.42	(0.22, 54.04)	0.3827	
	SoC	5	1 (20.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	9 (47.4)	26.14	(12.14, NE)	0.66	(0.19, 2.27)	0.5107	
	SoC	10	4 (40.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	3 (18.8)	NE	(12.14, NE)	NE	(NE, NE)	NE	
	SoC	8	1 (12.5)	NE	(20.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								0.2675

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	4 (33.3)	NE	(20.14, NE)	0.11	(0.01, 2.02)	0.1359	
	SoC	2	1 (50.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	8 (34.8)	NE	(12.14, NE)	0.97	(0.28, 3.32)	0.9559	
	SoC	16	4 (25.0)	NE	(12.29, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								0.5545

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	7 (33.3)	NE	(12.14, NE)	0.59	(0.15, 2.28)	0.4400	
	SoC	8	3 (37.5)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QIWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Insomnia (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	5 (35.7)	NE	(4.29, NE)	1.09	(0.19, 6.13)	0.9206	
	SoC	10	2 (20.0)	20.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QAWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								0.6279

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QAWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored) by age ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	8 (27.6)	NE	(26.14, NE)	0.33	(0.11, 0.99)	0.0471	*
	SoC	13	6 (46.2)	18.29	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QAWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored) by age ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	1 (16.7)	NE	(4.29, NE)	0.26	(0.02, 4.20)	0.3404	
	SoC	5	2 (40.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QAWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.6276

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QAWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	6 (31.6)	NE	(20.43, NE)	0.20	(0.05, 0.80)	0.0227	*
	SoC	10	5 (50.0)	16.43	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QAWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	3 (18.8)	NE	(12.14, NE)	0.21	(0.02, 2.08)	0.1825	
	SoC	8	3 (37.5)	20.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QAWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QAWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	4 (33.3)	NE	(8.14, NE)	NE	(NE, NE)	NE	
	SoC	2	1 (50.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QAWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	5 (21.7)	NE	(NE, NE)	0.26	(0.08, 0.88)	0.0297	*
	SoC	16	7 (43.8)	20.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QAWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								0.4178

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QAWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	3 (14.3)	NE	(NE, NE)	0.17	(0.03, 0.88)	0.0352	*
	SoC	8	3 (37.5)	16.43	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QAWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Appetite Loss (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	6 (42.9)	NE	(8.14, NE)	0.47	(0.14, 1.64)	0.2391	
	SoC	10	5 (50.0)	20.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QCWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Constipation (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Constipation (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QCWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Constipation (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Constipation (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	8 (27.6)	NE	(NE, NE)	0.50	(0.16, 1.57)	0.2339	
	SoC	13	5 (38.5)	20.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QCWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Constipation (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Constipation (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	3 (50.0)	20.14	(4.57, NE)	NE	(NE, NE)	NE	
	SoC	5	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QCWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Constipation (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Constipation (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								0.1895

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QCWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Constipation (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Constipation (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	3 (25.0)	NE	(4.57, NE)	0.35	(0.03, 3.69)	0.3857	
	SoC	2	1 (50.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QCWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Constipation (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Constipation (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	8 (34.8)	NE	(16.29, NE)	0.88	(0.26, 3.02)	0.8435	
	SoC	16	4 (25.0)	NE	(8.29, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QHWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Diarrhoea (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Diarrhoea (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QHWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Diarrhoea (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Diarrhoea (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	8 (27.6)	NE	(NE, NE)	0.44	(0.13, 1.52)	0.1950	
	SoC	13	4 (30.8)	12.29	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QHWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Diarrhoea (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Diarrhoea (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	2 (33.3)	NE	(4.29, NE)	NE	(NE, NE)	NE	
	SoC	5	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QHWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Diarrhoea (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Diarrhoea (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QHWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Diarrhoea (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Diarrhoea (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	2 (16.7)	NE	(16.14, NE)	NE	(NE, NE)	NE	
	SoC	2	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QHWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Diarrhoea (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Diarrhoea (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	8 (34.8)	NE	(12.14, NE)	0.88	(0.26, 3.00)	0.8351	
	SoC	16	4 (25.0)	NE	(8.29, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QGWC_SICC: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA				
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA	
Age	Peg	Overall									0.5592

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SICC: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 60	Peg	Week 4	29	26 (89.7)	15.54 (2.67)	(10.11, 20.97)	13.69 (4.95)	(3.62, 23.77)	0.0092	*
		Week 8	29	24 (82.8)	16.84 (3.26)	(10.19, 23.48)	14.89 (5.88)	(2.90, 26.89)	0.0166	*
		Week 12	29	26 (89.7)	16.02 (3.32)	(9.26, 22.77)	15.73 (6.17)	(3.18, 28.28)	0.0156	*
		Week 16	29	25 (86.2)	15.61 (3.35)	(8.79, 22.44)	10.47 (5.91)	(-1.56, 22.50)	0.0858	
		Week 20	29	27 (93.1)	17.84 (2.63)	(12.50, 23.19)	18.45 (4.95)	(8.39, 28.51)	0.0007	*
		Week 26	29	23 (79.3)	24.58 (3.36)	(17.70, 31.45)	23.97 (6.32)	(11.05, 36.89)	0.0007	*
	SoC	Week 4	13	11 (84.6)	1.85 (4.12)	(-6.53, 10.22)				
		Week 8	13	11 (84.6)	1.94 (4.82)	(-7.90, 11.78)				
		Week 12	13	11 (84.6)	0.29 (5.12)	(-10.14, 10.71)				
		Week 16	13	12 (92.3)	5.14 (4.84)	(-4.70, 14.98)				
		Week 20	13	11 (84.6)	-0.61 (4.15)	(-9.05, 7.83)				
		Week 26	13	10 (76.9)	0.60 (5.23)	(-10.10, 11.30)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SICC: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	4.35 (6.23)	(-11.66, 20.37)	9.22 (10.10)	(-16.74, 35.18)	0.4030	
		Week 8	6	5 (83.3)	16.57 (5.34)	(2.85, 30.29)	36.86 (8.65)	(14.63, 59.09)	0.0080	*
		Week 12	6	5 (83.3)	7.90 (6.72)	(-9.37, 25.18)	32.16 (10.89)	(4.16, 60.15)	0.0318	*
		Week 16	6	5 (83.3)	13.53 (5.98)	(-1.84, 28.90)	30.43 (9.57)	(5.83, 55.03)	0.0245	*
		Week 20	6	5 (83.3)	12.17 (4.94)	(-0.54, 24.88)	26.51 (7.91)	(6.17, 46.85)	0.0203	*
	Week 26	6	4 (66.7)	20.08 (6.13)	(-6.30, 46.46)	28.03 (11.59)	(-21.83, 77.89)	0.1367		
	SoC	Week 4	5	4 (80.0)	-4.87 (7.05)	(-23.00, 13.26)				
		Week 8	5	4 (80.0)	-20.29 (6.04)	(-35.82, -4.76)				
		Week 12	5	4 (80.0)	-24.26 (7.61)	(-43.81, -4.70)				
		Week 16	5	4 (80.0)	-16.90 (6.87)	(-34.57, 0.77)				
		Week 20	5	4 (80.0)	-14.35 (5.68)	(-28.96, 0.27)				
Week 26	5	2 (40.0)	-7.95 (9.36)	(-48.21, 32.30)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SIHC: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Global Health Status/QoL (censored)	< 60	Baseline	Peg	29	29 (100.0)	60.92 (19.68)	25.0	66.67	100.0	
			SoC	13	12 (92.3)	62.50 (17.94)	33.3	66.67	83.3	
		Week 4	Peg	29	26 (89.7)	75.96 (12.76)	50.0	79.17	100.0	
			SoC	13	12 (92.3)	63.89 (16.79)	33.3	66.67	83.3	
		Week 8	Peg	29	24 (82.8)	77.78 (16.79)	50.0	83.33	100.0	
			SoC	13	11 (84.6)	62.88 (17.23)	33.3	66.67	83.3	
		Week 12	Peg	29	26 (89.7)	76.92 (18.30)	33.3	83.33	100.0	
			SoC	13	11 (84.6)	62.12 (17.23)	33.3	66.67	83.3	
		Week 16	Peg	29	25 (86.2)	78.00 (15.38)	41.7	83.33	100.0	
			SoC	13	12 (92.3)	68.06 (22.98)	33.3	66.67	100.0	
		Week 20	Peg	29	27 (93.1)	79.01 (12.53)	50.0	83.33	100.0	
			SoC	13	12 (92.3)	63.19 (17.93)	33.3	66.67	83.3	
		Week 26	Peg	29	23 (79.3)	84.06 (15.27)	50.0	83.33	100.0	
			SoC	13	10 (76.9)	61.67 (19.33)	33.3	66.67	83.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SIHC: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Global Health Status/QoL (censored)	>= 60	Baseline	Peg	6	6 (100.0)	75.00 (16.67)	50.0	75.00	100.0	
			SoC	5	4 (80.0)	60.42 (7.98)	50.0	62.50	66.7	
		Week 4	Peg	6	5 (83.3)	76.67 (19.00)	50.0	83.33	100.0	
			SoC	5	5 (100.0)	63.33 (15.14)	41.7	66.67	83.3	
		Week 8	Peg	6	5 (83.3)	83.33 (16.67)	66.7	83.33	100.0	
			SoC	5	5 (100.0)	56.67 (14.91)	50.0	50.00	83.3	
		Week 12	Peg	6	5 (83.3)	76.67 (13.69)	66.7	75.00	100.0	
			SoC	5	5 (100.0)	53.33 (18.26)	33.3	50.00	83.3	
		Week 16	Peg	6	5 (83.3)	80.00 (13.94)	66.7	83.33	100.0	
			SoC	5	5 (100.0)	55.00 (16.24)	41.7	50.00	83.3	
		Week 20	Peg	6	5 (83.3)	80.00 (13.94)	66.7	83.33	100.0	
			SoC	5	5 (100.0)	55.00 (16.24)	41.7	50.00	83.3	
		Week 26	Peg	6	4 (66.7)	83.33 (13.61)	66.7	83.33	100.0	
			SoC	5	3 (60.0)	61.11 (19.25)	50.0	50.00	83.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SIHC: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in QLQ-C30 Global Health Status/QoL (censored)	Week 4	Peg	29	26 (89.7)	15.71 (18.61)	-8.3	12.50	50.0	0.75 (0.38) [0.05, 1.55]
		SoC	13	11 (84.6)	1.52 (18.19)	-25.0	0.00	33.3	
	Week 8	Peg	29	24 (82.8)	16.67 (19.66)	-16.7	16.67	66.7	0.76 (0.39) [0.05, 1.58]
		SoC	13	11 (84.6)	2.27 (15.41)	-25.0	0.00	33.3	
	Week 12	Peg	29	26 (89.7)	15.38 (20.51)	-16.7	16.67	50.0	0.67 (0.38) [-0.04, 1.46]
		SoC	13	11 (84.6)	1.52 (19.66)	-25.0	8.33	33.3	
	Week 16	Peg	29	25 (86.2)	15.33 (19.79)	-16.7	8.33	66.7	0.49 (0.37) [-0.20, 1.24]
		SoC	13	12 (92.3)	5.56 (18.91)	-25.0	4.17	33.3	
	Week 20	Peg	29	27 (93.1)	17.28 (20.27)	-16.7	16.67	66.7	0.82 (0.38) [0.12, 1.63]
		SoC	13	11 (84.6)	0.76 (18.05)	-25.0	0.00	33.3	
	Week 26	Peg	29	23 (79.3)	23.55 (22.14)	-16.7	25.00	66.7	0.93 (0.41) [0.19, 1.81]
		SoC	13	10 (76.9)	3.33 (18.51)	-25.0	4.17	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SIHC: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in QLQ-C30 Global Health Status/QoL (censored)	Week 4	Peg	6	5 (83.3)	0.00 (13.18)	-16.7	0.00	16.7	0.12 (0.80) [-1.42, 1.76]
		SoC	5	4 (80.0)	-2.08 (17.18)	-25.0	0.00	16.7	
	Week 8	Peg	6	5 (83.3)	6.67 (27.26)	-33.3	16.67	33.3	0.71 (0.84) [-0.67, 2.65]
		SoC	5	4 (80.0)	-10.42 (7.98)	-16.7	-12.50	0.0	
	Week 12	Peg	6	5 (83.3)	0.00 (22.82)	-33.3	0.00	25.0	0.66 (0.83) [-0.73, 2.56]
		SoC	5	4 (80.0)	-14.58 (14.23)	-33.3	-12.50	0.0	
	Week 16	Peg	6	5 (83.3)	10.00 (16.03)	-8.3	8.33	33.3	1.43 (0.95) [0.11, 3.82]
		SoC	5	4 (80.0)	-12.50 (10.76)	-25.0	-12.50	0.0	
	Week 20	Peg	6	5 (83.3)	10.00 (10.87)	-8.3	16.67	16.7	1.85 (1.04) [0.52, 4.56]
		SoC	5	4 (80.0)	-12.50 (10.76)	-25.0	-12.50	0.0	
	Week 26	Peg	6	4 (66.7)	12.50 (17.35)	-8.3	12.50	33.3	0.87 (1.35) [-1.01, 4.16]
		SoC	5	2 (40.0)	-4.17 (5.89)	-8.3	-4.17	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SICD: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.2574

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SICD: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Male	Peg	Week 4	19	16 (84.2)	15.18 (3.77)	(7.30, 23.05)	16.04 (6.57)	(2.34, 29.75)	0.0240	*
		Week 8	19	15 (78.9)	15.06 (4.40)	(5.85, 24.26)	18.83 (7.61)	(2.91, 34.75)	0.0229	*
		Week 12	19	17 (89.5)	11.53 (4.70)	(1.76, 21.30)	20.49 (8.43)	(2.96, 38.02)	0.0241	*
		Week 16	19	16 (84.2)	10.09 (3.93)	(1.91, 18.27)	12.39 (6.68)	(-1.51, 26.28)	0.0778	
		Week 20	19	17 (89.5)	15.80 (3.16)	(9.24, 22.36)	23.43 (5.63)	(11.71, 35.14)	0.0004	*
	Week 26	19	14 (73.7)	22.55 (4.48)	(13.05, 32.05)	27.96 (8.47)	(9.99, 45.92)	0.0045	*	
	SoC	Week 4	10	8 (80.0)	-0.87 (5.39)	(-12.11, 10.37)				
		Week 8	10	8 (80.0)	-3.77 (6.14)	(-16.63, 9.08)				
		Week 12	10	8 (80.0)	-8.96 (6.95)	(-23.40, 5.49)				
		Week 16	10	9 (90.0)	-2.30 (5.37)	(-13.45, 8.86)				
		Week 20	10	8 (80.0)	-7.63 (4.65)	(-17.30, 2.04)				
Week 26	10	6 (60.0)	-5.40 (6.99)	(-20.21, 9.41)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SICD: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Female	Peg	Week 4	16	15 (93.8)	10.58 (3.04)	(4.20, 16.96)	5.31 (6.41)	(-8.16, 18.78)	0.4186	
		Week 8	16	14 (87.5)	14.94 (4.15)	(6.18, 23.70)	11.09 (8.72)	(-7.31, 29.49)	0.2208	
		Week 12	16	14 (87.5)	15.92 (3.16)	(9.25, 22.59)	13.32 (6.64)	(-0.70, 27.33)	0.0612	
		Week 16	16	14 (87.5)	20.59 (4.24)	(11.64, 29.53)	19.38 (8.91)	(0.59, 38.17)	0.0439	*
		Week 20	16	15 (93.8)	16.87 (2.85)	(10.89, 22.86)	14.73 (6.01)	(2.09, 27.36)	0.0248	*
		Week 26	16	13 (81.3)	22.71 (3.81)	(14.58, 30.84)	17.00 (8.11)	(-0.27, 34.28)	0.0533	
	SoC	Week 4	8	7 (87.5)	5.27 (5.31)	(-5.88, 16.42)				
		Week 8	8	7 (87.5)	3.86 (6.99)	(-10.90, 18.61)				
		Week 12	8	7 (87.5)	2.60 (5.33)	(-8.63, 13.84)				
		Week 16	8	7 (87.5)	1.21 (7.14)	(-13.86, 16.27)				
		Week 20	8	7 (87.5)	2.15 (4.98)	(-8.32, 12.61)				
		Week 26	8	6 (75.0)	5.70 (6.66)	(-8.49, 19.90)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SIHD: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Global Health Status/QoL (censored)	Male	Baseline	Peg	19	19 (100.0)	65.35 (20.65)	33.3	66.67	100.0	
			SoC	10	9 (90.0)	59.26 (18.37)	33.3	58.33	83.3	
		Week 4	Peg	19	16 (84.2)	76.56 (16.16)	50.0	79.17	100.0	
			SoC	10	9 (90.0)	61.11 (17.18)	33.3	66.67	83.3	
		Week 8	Peg	19	15 (78.9)	77.78 (18.81)	50.0	83.33	100.0	
			SoC	10	9 (90.0)	58.33 (17.68)	33.3	50.00	83.3	
		Week 12	Peg	19	17 (89.5)	74.51 (22.91)	33.3	75.00	100.0	
			SoC	10	9 (90.0)	53.70 (16.72)	33.3	50.00	83.3	
		Week 16	Peg	19	16 (84.2)	73.96 (16.63)	41.7	70.83	100.0	
			SoC	10	10 (100.0)	60.83 (22.92)	33.3	58.33	100.0	
		Week 20	Peg	19	17 (89.5)	77.45 (15.52)	50.0	75.00	100.0	
			SoC	10	9 (90.0)	54.63 (17.24)	33.3	50.00	83.3	
		Week 26	Peg	19	14 (73.7)	82.74 (18.04)	50.0	83.33	100.0	
			SoC	10	7 (70.0)	54.76 (18.54)	33.3	50.00	83.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SIHD: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Global Health Status/QoL (censored)	Female	Baseline	Peg	16	16 (100.0)	60.94 (18.93)	25.0	66.67	83.3	
			SoC	8	7 (87.5)	65.48 (12.20)	50.0	66.67	83.3	
		Week 4	Peg	16	15 (93.8)	75.56 (10.67)	58.3	83.33	91.7	
			SoC	8	8 (100.0)	66.67 (14.77)	41.7	66.67	83.3	
		Week 8	Peg	16	14 (87.5)	79.76 (14.51)	50.0	83.33	100.0	
			SoC	8	7 (87.5)	64.29 (15.00)	50.0	66.67	83.3	
		Week 12	Peg	16	14 (87.5)	79.76 (6.30)	66.7	83.33	83.3	
			SoC	8	7 (87.5)	66.67 (16.67)	50.0	66.67	83.3	
		Week 16	Peg	16	14 (87.5)	83.33 (11.32)	66.7	83.33	100.0	
			SoC	8	7 (87.5)	69.05 (20.25)	50.0	66.67	100.0	
		Week 20	Peg	16	15 (93.8)	81.11 (8.01)	66.7	83.33	100.0	
			SoC	8	8 (100.0)	67.71 (15.71)	50.0	70.83	83.3	
		Week 26	Peg	16	13 (81.3)	85.26 (10.84)	66.7	83.33	100.0	
			SoC	8	6 (75.0)	69.44 (16.39)	50.0	75.00	83.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SIHD: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Global Health Status/QoL (censored)	Male	Week 4	Peg	19	16 (84.2)	14.06 (20.35)	-16.7	16.67	50.0	0.62 (0.47) [-0.24, 1.60]
			SoC	10	8 (80.0)	2.08 (14.60)	-16.7	0.00	33.3	
		Week 8	Peg	19	15 (78.9)	13.33 (23.10)	-33.3	16.67	66.7	0.71 (0.48) [-0.15, 1.73]
			SoC	10	8 (80.0)	-1.04 (8.26)	-16.7	0.00	8.3	
		Week 12	Peg	19	17 (89.5)	9.80 (23.61)	-33.3	16.67	50.0	0.71 (0.46) [-0.14, 1.69]
			SoC	10	8 (80.0)	-6.25 (17.68)	-33.3	-4.17	16.7	
		Week 16	Peg	19	16 (84.2)	9.38 (17.71)	-16.7	4.17	50.0	0.60 (0.45) [-0.22, 1.54]
			SoC	10	9 (90.0)	-0.93 (14.10)	-25.0	0.00	16.7	
		Week 20	Peg	19	17 (89.5)	14.71 (16.80)	-8.3	16.67	50.0	1.20 (0.49) [0.36, 2.28]
			SoC	10	8 (80.0)	-5.21 (14.04)	-25.0	-4.17	16.7	
		Week 26	Peg	19	14 (73.7)	20.24 (22.10)	-8.3	16.67	66.7	0.98 (0.55) [0.02, 2.19]
			SoC	10	6 (60.0)	0.00 (11.79)	-16.7	0.00	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SIHD: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Global Health Status/QoL (censored)	Female	Week 4	Peg	16	15 (93.8)	12.22 (17.21)	-8.3	8.33	50.0	0.70 (0.50) [-0.20, 1.76]
			SoC	8	7 (87.5)	-1.19 (21.21)	-25.0	0.00	25.0	
		Week 8	Peg	16	14 (87.5)	16.67 (19.06)	-16.7	16.67	50.0	0.88 (0.51) [-0.03, 2.00]
			SoC	8	7 (87.5)	-1.19 (20.65)	-25.0	0.00	33.3	
		Week 12	Peg	16	14 (87.5)	16.67 (18.20)	0.0	12.50	50.0	0.77 (0.51) [-0.14, 1.87]
			SoC	8	7 (87.5)	1.19 (21.75)	-25.0	0.00	33.3	
		Week 16	Peg	16	14 (87.5)	20.24 (19.53)	-8.3	20.83	66.7	0.75 (0.51) [-0.16, 1.85]
			SoC	8	7 (87.5)	3.57 (24.47)	-25.0	0.00	33.3	
		Week 20	Peg	16	15 (93.8)	17.78 (22.02)	-16.7	16.67	66.7	0.79 (0.50) [-0.11, 1.87]
			SoC	8	7 (87.5)	0.00 (20.97)	-25.0	0.00	33.3	
		Week 26	Peg	16	13 (81.3)	23.72 (21.74)	-16.7	25.00	66.7	0.85 (0.55) [-0.12, 2.06]
			SoC	8	6 (75.0)	4.17 (22.20)	-25.0	8.33	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SICB: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.5703

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SICB: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Latin America	Peg	Week 4	12	11 (91.7)	25.75 (2.97)	(19.04, 32.46)	17.80 (7.58)	(0.65, 34.94)	0.0435	*
		Week 8	12	10 (83.3)	28.78 (3.11)	(21.60, 35.96)	22.68 (7.63)	(5.07, 40.28)	0.0179	*
		Week 12	12	11 (91.7)	21.99 (5.44)	(9.68, 34.29)	14.89 (13.90)	(-16.54, 46.33)	0.3117	
		Week 16	12	10 (83.3)	22.07 (4.76)	(11.09, 33.04)	15.74 (11.68)	(-11.19, 42.67)	0.2148	
		Week 20	12	10 (83.3)	28.37 (3.50)	(20.30, 36.43)	20.19 (8.66)	(0.23, 40.16)	0.0480	*
		Week 26	12	9 (75.0)	34.95 (3.13)	(27.55, 42.36)	25.96 (7.38)	(8.52, 43.40)	0.0097	*
	SoC	Week 4	2	2 (100.0)	7.96 (6.97)	(-7.80, 23.71)				
		Week 8	2	2 (100.0)	6.10 (6.97)	(-9.96, 22.17)				
		Week 12	2	2 (100.0)	7.09 (12.77)	(-21.80, 35.98)				
		Week 16	2	2 (100.0)	6.33 (10.66)	(-18.25, 30.91)				
		Week 20	2	2 (100.0)	8.17 (7.89)	(-10.01, 26.36)				
		Week 26	2	2 (100.0)	9.00 (6.66)	(-6.77, 24.76)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SICB: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Asia Pacific Peg	Peg	Week 4	23	20 (87.0)	6.49 (2.84)	(0.68, 12.30)	7.27 (4.70)	(-2.33, 16.88)	0.1324	
		Week 8	23	19 (82.6)	8.64 (3.54)	(1.38, 15.90)	12.30 (5.77)	(0.48, 24.13)	0.0420	*
		Week 12	23	20 (87.0)	10.36 (3.62)	(2.95, 17.77)	19.00 (6.03)	(6.67, 31.32)	0.0037	*
		Week 16	23	20 (87.0)	11.61 (3.68)	(4.08, 19.13)	12.72 (5.87)	(0.73, 24.70)	0.0383	*
		Week 20	23	22 (95.7)	12.17 (2.89)	(6.27, 18.06)	19.46 (4.94)	(9.38, 29.54)	0.0004	*
	Week 26	23	18 (78.3)	17.00 (3.75)	(9.27, 24.73)	20.51 (6.90)	(6.26, 34.75)	0.0067	*	
	SoC	Week 4	16	13 (81.3)	-0.79 (3.60)	(-8.14, 6.57)				
		Week 8	16	13 (81.3)	-3.66 (4.34)	(-12.54, 5.22)				
		Week 12	16	13 (81.3)	-8.63 (4.60)	(-18.04, 0.77)				
		Week 16	16	14 (87.5)	-1.11 (4.43)	(-10.16, 7.95)				
Week 20		16	13 (81.3)	-7.29 (3.85)	(-15.15, 0.57)					
Week 26	16	10 (62.5)	-3.50 (5.51)	(-14.87, 7.86)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SIHB: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Global Health Status/QoL (censored)	Latin America	Baseline	Peg	12	12 (100.0)	54.86 (19.93)	25.0	58.33	83.3	
			SoC	2	2 (100.0)	62.50 (5.89)	58.3	62.50	66.7	
		Week 4	Peg	12	11 (91.7)	84.09 (9.47)	66.7	83.33	100.0	
	SoC		2	2 (100.0)	66.67 (0.00)	66.7	66.67	66.7		
		Week 8	Peg	12	10 (83.3)	89.17 (9.66)	75.0	83.33	100.0	
	SoC		2	2 (100.0)	66.67 (0.00)	66.7	66.67	66.7		
		Week 12	Peg	12	11 (91.7)	80.30 (17.98)	33.3	83.33	100.0	
	SoC		2	2 (100.0)	66.67 (0.00)	66.7	66.67	66.7		
		Week 16	Peg	12	10 (83.3)	82.50 (14.41)	58.3	83.33	100.0	
	SoC		2	2 (100.0)	66.67 (0.00)	66.7	66.67	66.7		
		Week 20	Peg	12	10 (83.3)	85.00 (11.65)	66.7	83.33	100.0	
	SoC		2	2 (100.0)	66.67 (0.00)	66.7	66.67	66.7		
		Week 26	Peg	12	9 (75.0)	92.59 (8.78)	83.3	100.00	100.0	
	SoC		2	2 (100.0)	66.67 (0.00)	66.7	66.67	66.7		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SIHB: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Global Health Status/QoL (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	67.75 (18.52)	33.3	66.67	100.0	
			SoC	16	14 (87.5)	61.90 (16.89)	33.3	66.67	83.3	
	Week 4	Peg	23	20 (87.0)	71.67 (13.63)	50.0	66.67	100.0		
		SoC	16	15 (93.8)	63.33 (16.90)	33.3	66.67	83.3		
	Week 8	Peg	23	19 (82.6)	73.25 (17.03)	50.0	75.00	100.0		
		SoC	16	14 (87.5)	60.12 (17.35)	33.3	50.00	83.3		
	Week 12	Peg	23	20 (87.0)	75.00 (17.31)	33.3	79.17	100.0		
		SoC	16	14 (87.5)	58.33 (18.49)	33.3	50.00	83.3		
	Week 16	Peg	23	20 (87.0)	76.25 (15.12)	41.7	79.17	100.0		
		SoC	16	15 (93.8)	63.89 (23.07)	33.3	50.00	100.0		
	Week 20	Peg	23	22 (95.7)	76.52 (12.24)	50.0	79.17	100.0		
		SoC	16	15 (93.8)	60.00 (18.42)	33.3	50.00	83.3		
	Week 26	Peg	23	18 (78.3)	79.63 (15.45)	50.0	83.33	100.0		
		SoC	16	11 (68.8)	60.61 (20.10)	33.3	50.00	83.3		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SIHB: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline in QLQ-C30 Global Health Status/QoL (censored)	Latin America	Week 4	Peg	12	11 (91.7)	26.52 (19.30)	0.0	33.33	50.0	1.12 (0.90)	[-0.40, 3.16]
			SoC	2	2 (100.0)	4.17 (5.89)	0.0	4.17	8.3		
		Week 8	Peg	12	10 (83.3)	29.17 (18.94)	0.0	29.17	66.7	1.28 (0.94)	[-0.25, 3.45]
			SoC	2	2 (100.0)	4.17 (5.89)	0.0	4.17	8.3		
		Week 12	Peg	12	11 (91.7)	22.73 (21.76)	-16.7	25.00	50.0	0.83 (0.88)	[-0.72, 2.76]
			SoC	2	2 (100.0)	4.17 (5.89)	0.0	4.17	8.3		
		Week 16	Peg	12	10 (83.3)	22.50 (23.26)	-16.7	16.67	66.7	0.76 (0.89)	[-0.81, 2.73]
			SoC	2	2 (100.0)	4.17 (5.89)	0.0	4.17	8.3		
		Week 20	Peg	12	10 (83.3)	29.17 (17.68)	8.3	25.00	66.7	1.37 (0.94)	[-0.16, 3.58]
			SoC	2	2 (100.0)	4.17 (5.89)	0.0	4.17	8.3		
		Week 26	Peg	12	9 (75.0)	36.11 (21.25)	8.3	33.33	66.7	1.45 (0.99)	[-0.08, 3.81]
			SoC	2	2 (100.0)	4.17 (5.89)	0.0	4.17	8.3		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SIHB: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Global Health Status/QoL (censored)	Asia Pacific	Week 4	Peg	23	20 (87.0)	5.83 (13.81)	-16.7	0.00	33.3	0.36 (0.37) [-0.35, 1.12]
			SoC	16	13 (81.3)	0.00 (18.63)	-25.0	0.00	33.3	
		Week 8	Peg	23	19 (82.6)	7.46 (18.19)	-33.3	8.33	50.0	0.53 (0.38) [-0.18, 1.32]
			SoC	16	13 (81.3)	-1.92 (15.65)	-25.0	0.00	33.3	
		Week 12	Peg	23	20 (87.0)	7.50 (19.48)	-33.3	4.17	50.0	0.56 (0.38) [-0.14, 1.34]
			SoC	16	13 (81.3)	-3.85 (20.59)	-33.3	-8.33	33.3	
		Week 16	Peg	23	20 (87.0)	10.42 (15.74)	-8.3	4.17	33.3	0.54 (0.37) [-0.14, 1.31]
			SoC	16	14 (87.5)	0.60 (20.01)	-25.0	0.00	33.3	
		Week 20	Peg	23	22 (95.7)	10.23 (17.05)	-16.7	4.17	50.0	0.79 (0.38) [0.10, 1.58]
			SoC	16	13 (81.3)	-3.85 (18.20)	-25.0	-8.33	33.3	
		Week 26	Peg	23	18 (78.3)	14.81 (18.43)	-16.7	12.50	50.0	0.69 (0.42) [-0.09, 1.58]
			SoC	16	10 (62.5)	1.67 (18.76)	-25.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SICA: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.5034

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SICA: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 4	21	18 (85.7)	16.45 (2.74)	(10.73, 22.17)	15.67 (5.89)	(3.38, 27.96)	0.0151	*
		Week 8	21	17 (81.0)	15.62 (3.98)	(7.29, 23.95)	18.73 (8.40)	(1.15, 36.31)	0.0380	*
		Week 12	21	18 (85.7)	14.17 (3.69)	(6.47, 21.87)	28.52 (7.94)	(11.96, 45.08)	0.0018	*
		Week 16	21	17 (81.0)	13.68 (4.17)	(4.99, 22.38)	13.57 (8.16)	(-3.46, 30.59)	0.1120	
		Week 20	21	18 (85.7)	18.53 (2.87)	(12.53, 24.52)	26.89 (6.17)	(14.02, 39.75)	0.0003	*
	Week 26	21	15 (71.4)	26.15 (3.93)	(17.78, 34.52)	31.89 (9.80)	(11.01, 52.77)	0.0053	*	
	SoC	Week 4	8	5 (62.5)	0.78 (5.21)	(-10.08, 11.65)				
		Week 8	8	5 (62.5)	-3.11 (7.37)	(-18.53, 12.32)				
		Week 12	8	5 (62.5)	-14.35 (7.02)	(-28.99, 0.29)				
		Week 16	8	6 (75.0)	0.12 (7.02)	(-14.52, 14.75)				
		Week 20	8	5 (62.5)	-8.36 (5.45)	(-19.74, 3.01)				
Week 26		8	3 (37.5)	-5.74 (8.91)	(-24.74, 13.25)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SICA: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	10.04 (4.17)	(1.33, 18.74)	11.42 (6.34)	(-1.80, 24.63)	0.0867	
		Week 8	14	12 (85.7)	15.71 (4.51)	(6.26, 25.16)	17.89 (6.70)	(3.86, 31.92)	0.0152	*
		Week 12	14	13 (92.9)	13.09 (4.65)	(3.39, 22.80)	12.61 (7.10)	(-2.20, 27.42)	0.0909	
		Week 16	14	13 (92.9)	16.34 (4.50)	(6.95, 25.73)	15.92 (6.87)	(1.59, 30.24)	0.0312	*
		Week 20	14	14 (100.0)	14.56 (3.65)	(6.97, 22.16)	16.62 (5.70)	(4.78, 28.47)	0.0082	*
	Week 26	14	12 (85.7)	20.19 (4.52)	(10.68, 29.69)	20.25 (7.00)	(5.56, 34.95)	0.0096	*	
	SoC	Week 4	10	10 (100.0)	-1.38 (4.76)	(-11.31, 8.55)				
		Week 8	10	10 (100.0)	-2.18 (4.95)	(-12.53, 8.17)				
		Week 12	10	10 (100.0)	0.48 (5.31)	(-10.60, 11.56)				
		Week 16	10	10 (100.0)	0.42 (5.14)	(-10.30, 11.15)				
		Week 20	10	10 (100.0)	-2.06 (4.33)	(-11.06, 6.95)				
	Week 26	10	9 (90.0)	-0.07 (5.24)	(-11.08, 10.95)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SIHA: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Global Health Status/QoL (censored)	< 4	Baseline	Peg	21	21 (100.0)	61.51 (22.28)	25.0	66.67	100.0	
			SoC	8	6 (75.0)	62.50 (19.54)	33.3	62.50	83.3	
		Week 4	Peg	21	18 (85.7)	78.24 (11.12)	58.3	83.33	100.0	
			SoC	8	7 (87.5)	67.86 (16.96)	33.3	66.67	83.3	
		Week 8	Peg	21	17 (81.0)	78.92 (17.46)	50.0	83.33	100.0	
			SoC	8	6 (75.0)	62.50 (18.07)	41.7	58.33	83.3	
		Week 12	Peg	21	18 (85.7)	75.93 (15.89)	33.3	79.17	100.0	
			SoC	8	6 (75.0)	52.78 (20.18)	33.3	50.00	83.3	
		Week 16	Peg	21	17 (81.0)	75.98 (14.99)	41.7	75.00	100.0	
			SoC	8	7 (87.5)	65.48 (24.73)	33.3	66.67	100.0	
		Week 20	Peg	21	18 (85.7)	78.70 (11.15)	58.3	83.33	100.0	
			SoC	8	7 (87.5)	60.71 (19.67)	33.3	66.67	83.3	
		Week 26	Peg	21	15 (71.4)	82.22 (14.39)	58.3	83.33	100.0	
			SoC	8	4 (50.0)	58.33 (21.52)	33.3	58.33	83.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SIHA: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Global Health Status/QoL (censored)	>= 4	Baseline	Peg	14	14 (100.0)	66.07 (15.49)	50.0	66.67	100.0	
			SoC	10	10 (100.0)	61.67 (14.27)	33.3	66.67	83.3	
		Week 4	Peg	14	13 (92.9)	73.08 (16.37)	50.0	66.67	100.0	
			SoC	10	10 (100.0)	60.83 (15.24)	33.3	66.67	83.3	
		Week 8	Peg	14	12 (85.7)	78.47 (16.07)	50.0	83.33	100.0	
			SoC	10	10 (100.0)	60.00 (16.10)	33.3	58.33	83.3	
		Week 12	Peg	14	13 (92.9)	78.21 (19.99)	33.3	83.33	100.0	
			SoC	10	10 (100.0)	63.33 (15.32)	50.0	58.33	83.3	
		Week 16	Peg	14	13 (92.9)	81.41 (14.89)	50.0	83.33	100.0	
			SoC	10	10 (100.0)	63.33 (20.49)	33.3	58.33	100.0	
		Week 20	Peg	14	14 (100.0)	79.76 (14.51)	50.0	83.33	100.0	
			SoC	10	10 (100.0)	60.83 (16.69)	33.3	58.33	83.3	
		Week 26	Peg	14	12 (85.7)	86.11 (15.62)	50.0	83.33	100.0	
			SoC	10	9 (90.0)	62.96 (18.22)	33.3	66.67	83.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SIHA: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in QLQ-C30 Global Health Status/QoL (censored)	Week 4	Peg	21	18 (85.7)	15.74 (20.59)	-16.7	12.50	50.0	0.59 (0.54) [-0.42, 1.72]
		SoC	8	5 (62.5)	3.33 (19.18)	-16.7	0.00	33.3	
	Week 8	Peg	21	17 (81.0)	14.71 (23.48)	-33.3	8.33	66.7	0.66 (0.55) [-0.35, 1.82]
		SoC	8	5 (62.5)	0.00 (10.21)	-16.7	0.00	8.3	
	Week 12	Peg	21	18 (85.7)	13.43 (24.28)	-33.3	12.50	50.0	1.03 (0.56) [0.04, 2.25]
		SoC	8	5 (62.5)	-11.67 (19.18)	-33.3	-16.67	8.3	
	Week 16	Peg	21	17 (81.0)	13.73 (22.04)	-16.7	8.33	66.7	0.63 (0.51) [-0.31, 1.71]
		SoC	8	6 (75.0)	0.00 (17.48)	-25.0	4.17	16.7	
	Week 20	Peg	21	18 (85.7)	18.06 (22.91)	-16.7	12.50	66.7	1.08 (0.56) [0.09, 2.31]
		SoC	8	5 (62.5)	-6.67 (18.07)	-25.0	-16.67	16.7	
	Week 26	Peg	21	15 (71.4)	24.44 (25.09)	-16.7	16.67	66.7	0.85 (0.70) [-0.40, 2.36]
		SoC	8	3 (37.5)	2.78 (17.35)	-16.7	8.33	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWC_SIHA: Change from baseline in QLQ-C30 Global Health Status/QoL (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in QLQ-C30 Global Health Status/QoL (censored)	Week 4	Peg	14	13 (92.9)	9.62 (15.53)	-8.3	0.00	33.3	0.62 (0.45) [-0.21, 1.58]
		SoC	10	10 (100.0)	-0.83 (17.32)	-25.0	0.00	25.0	
	Week 8	Peg	14	12 (85.7)	15.28 (17.71)	-16.7	16.67	33.3	0.94 (0.48) [0.10, 1.99]
		SoC	10	10 (100.0)	-1.67 (17.03)	-25.0	0.00	33.3	
	Week 12	Peg	14	13 (92.9)	12.18 (17.22)	-16.7	16.67	33.3	0.57 (0.45) [-0.27, 1.52]
		SoC	10	10 (100.0)	1.67 (18.76)	-25.0	0.00	33.3	
	Week 16	Peg	14	13 (92.9)	15.38 (15.16)	-8.3	16.67	33.3	0.75 (0.46) [-0.07, 1.74]
		SoC	10	10 (100.0)	1.67 (20.34)	-25.0	0.00	33.3	
	Week 20	Peg	14	14 (100.0)	13.69 (13.32)	-8.3	16.67	33.3	0.93 (0.46) [0.12, 1.93]
		SoC	10	10 (100.0)	-0.83 (17.32)	-25.0	0.00	33.3	
	Week 26	Peg	14	12 (85.7)	18.75 (16.71)	-8.3	25.00	33.3	0.94 (0.50) [0.07, 2.03]
		SoC	10	9 (90.0)	1.85 (18.06)	-25.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SICC: Change from baseline in QLQ-C30 Physical Functioning (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.3798

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SICC: Change from baseline in QLQ-C30 Physical Functioning (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 60	Peg	Week 4	29	26 (89.7)	8.67 (1.84)	(4.92, 12.42)	11.03 (3.43)	(4.05, 18.01)	0.0029	*
		Week 8	29	24 (82.8)	9.62 (2.34)	(4.85, 14.40)	11.32 (4.25)	(2.65, 19.99)	0.0122	*
		Week 12	29	26 (89.7)	10.63 (2.23)	(6.08, 15.17)	11.50 (4.16)	(3.03, 19.96)	0.0093	*
		Week 16	29	25 (86.2)	11.54 (2.69)	(6.07, 17.02)	11.22 (4.75)	(1.56, 20.88)	0.0242	*
		Week 20	29	27 (93.1)	10.65 (2.13)	(6.32, 14.97)	11.32 (4.01)	(3.17, 19.48)	0.0079	*
		Week 26	29	23 (79.3)	11.14 (3.06)	(4.88, 17.40)	12.16 (5.72)	(0.46, 23.86)	0.0422	*
	SoC	Week 4	13	11 (84.6)	-2.36 (2.85)	(-8.16, 3.44)				
		Week 8	13	11 (84.6)	-1.69 (3.48)	(-8.79, 5.40)				
		Week 12	13	11 (84.6)	-0.87 (3.45)	(-7.90, 6.16)				
		Week 16	13	12 (92.3)	0.33 (3.88)	(-7.57, 8.23)				
		Week 20	13	11 (84.6)	-0.68 (3.36)	(-7.51, 6.16)				
		Week 26	13	10 (76.9)	-1.02 (4.74)	(-10.72, 8.67)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SICC: Change from baseline in QLQ-C30 Physical Functioning (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	9.02 (12.91)	(-24.16, 42.19)	36.42 (21.07)	(-17.75, 90.59)	0.1445	
		Week 8	6	5 (83.3)	8.34 (12.31)	(-23.30, 39.98)	28.66 (20.10)	(-23.01, 80.32)	0.2132	
		Week 12	6	5 (83.3)	2.25 (12.74)	(-30.50, 35.00)	21.67 (20.80)	(-31.80, 75.15)	0.3452	
		Week 16	6	5 (83.3)	7.49 (11.71)	(-22.60, 37.58)	25.24 (18.52)	(-22.37, 72.86)	0.2311	
		Week 20	6	5 (83.3)	3.57 (12.07)	(-27.45, 34.59)	20.48 (19.09)	(-28.61, 69.56)	0.3326	
		Week 26	6	4 (66.7)	4.54 (3.11)	(-8.84, 17.93)	-4.51 (5.74)	(-29.21, 20.20)	0.5145	
	SoC	Week 4	5	4 (80.0)	-27.40 (14.68)	(-65.14, 10.34)				
		Week 8	5	4 (80.0)	-20.32 (14.00)	(-56.32, 15.67)				
		Week 12	5	4 (80.0)	-19.42 (14.49)	(-56.68, 17.83)				
		Week 16	5	4 (80.0)	-17.75 (13.72)	(-53.02, 17.52)				
		Week 20	5	4 (80.0)	-16.90 (14.14)	(-53.26, 19.45)				
		Week 26	5	2 (40.0)	9.05 (4.91)	(-12.08, 30.19)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SIHC: Change from baseline in QLQ-C30 Physical Functioning (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Physical Functioning (censored)	< 60	Baseline	Peg	29	29 (100.0)	79.77 (14.96)	40.0	86.67	100.0	
			SoC	13	12 (92.3)	79.44 (20.59)	26.7	86.67	100.0	
	Week 4	Peg	29	26 (89.7)	89.74 (10.87)	53.3	93.33	100.0		
		SoC	13	12 (92.3)	82.22 (14.02)	60.0	80.00	100.0		
	Week 8	Peg	29	24 (82.8)	90.56 (11.45)	53.3	93.33	100.0		
		SoC	13	11 (84.6)	80.61 (14.13)	60.0	80.00	100.0		
	Week 12	Peg	29	26 (89.7)	91.54 (11.40)	60.0	93.33	100.0		
		SoC	13	11 (84.6)	81.82 (14.33)	53.3	80.00	100.0		
	Week 16	Peg	29	25 (86.2)	92.00 (14.53)	33.3	100.00	100.0		
		SoC	13	12 (92.3)	80.00 (16.58)	40.0	80.00	100.0		
	Week 20	Peg	29	27 (93.1)	92.10 (11.40)	53.3	93.33	100.0		
		SoC	13	12 (92.3)	83.33 (11.19)	66.7	80.00	100.0		
	Week 26	Peg	29	23 (79.3)	90.43 (15.02)	33.3	93.33	100.0		
		SoC	13	10 (76.9)	80.00 (14.05)	53.3	80.00	100.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SIHC: Change from baseline in QLQ-C30 Physical Functioning (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Physical Functioning (censored)	>= 60	Baseline	Peg	6	6 (100.0)	83.33 (21.81)	46.7	93.33	100.0	
			SoC	5	4 (80.0)	78.33 (3.33)	73.3	80.00	80.0	
	Week 4	Peg	6	5 (83.3)	93.33 (8.16)	80.0	93.33	100.0		
		SoC	5	5 (100.0)	65.33 (35.71)	6.7	66.67	100.0		
	Week 8	Peg	6	5 (83.3)	94.67 (5.58)	86.7	93.33	100.0		
		SoC	5	5 (100.0)	70.67 (32.18)	20.0	80.00	100.0		
	Week 12	Peg	6	5 (83.3)	89.33 (10.11)	80.0	86.67	100.0		
		SoC	5	5 (100.0)	70.67 (32.18)	20.0	80.00	100.0		
	Week 16	Peg	6	5 (83.3)	88.00 (11.93)	73.3	86.67	100.0		
		SoC	5	5 (100.0)	69.33 (32.86)	20.0	80.00	100.0		
	Week 20	Peg	6	5 (83.3)	84.00 (21.91)	46.7	86.67	100.0		
		SoC	5	5 (100.0)	70.67 (32.18)	20.0	80.00	100.0		
	Week 26	Peg	6	4 (66.7)	91.67 (10.00)	80.0	93.33	100.0		
		SoC	5	3 (60.0)	91.11 (10.18)	80.0	93.33	100.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SIHC: Change from baseline in QLQ-C30 Physical Functioning (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in QLQ-C30 Physical Functioning (censored)	Week 4	Peg	29	26 (89.7)	9.23 (11.17)	-20.0	13.33	26.7	1.17 (0.40) [0.46, 2.03]
		SoC	13	11 (84.6)	-3.64 (9.60)	-20.0	0.00	6.7	
	Week 8	Peg	29	24 (82.8)	10.56 (14.70)	-20.0	13.33	53.3	1.04 (0.40) [0.32, 1.89]
		SoC	13	11 (84.6)	-3.64 (9.60)	-20.0	0.00	6.7	
	Week 12	Peg	29	26 (89.7)	11.28 (14.33)	-13.3	13.33	53.3	1.02 (0.39) [0.31, 1.86]
		SoC	13	11 (84.6)	-2.42 (9.56)	-20.0	0.00	13.3	
	Week 16	Peg	29	25 (86.2)	11.47 (17.05)	-40.0	13.33	53.3	0.70 (0.37) [0.01, 1.48]
		SoC	13	12 (92.3)	0.56 (10.43)	-20.0	3.33	13.3	
	Week 20	Peg	29	27 (93.1)	11.36 (15.99)	-20.0	13.33	53.3	0.92 (0.39) [0.22, 1.74]
		SoC	13	11 (84.6)	-2.42 (10.44)	-20.0	0.00	13.3	
	Week 26	Peg	29	23 (79.3)	11.88 (18.31)	-40.0	13.33	53.3	0.87 (0.41) [0.13, 1.74]
		SoC	13	10 (76.9)	-2.67 (10.04)	-20.0	-3.33	13.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SIHC: Change from baseline in QLQ-C30 Physical Functioning (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in QLQ-C30 Physical Functioning (censored)	Week 4	Peg	6	5 (83.3)	2.67 (14.61)	-13.3	0.00	26.7	0.84 (0.85) [-0.52, 2.85]
		SoC	5	4 (80.0)	-21.67 (35.43)	-73.3	-10.00	6.7	
	Week 8	Peg	6	5 (83.3)	4.00 (8.94)	0.0	0.00	20.0	0.74 (0.84) [-0.64, 2.69]
		SoC	5	4 (80.0)	-15.00 (33.28)	-60.0	-6.67	13.3	
	Week 12	Peg	6	5 (83.3)	-1.33 (9.89)	-13.3	0.00	13.3	0.53 (0.82) [-0.89, 2.36]
		SoC	5	4 (80.0)	-15.00 (33.28)	-60.0	-6.67	13.3	
	Week 16	Peg	6	5 (83.3)	6.67 (13.33)	-6.7	0.00	26.7	0.85 (0.85) [-0.50, 2.87]
		SoC	5	4 (80.0)	-16.67 (33.77)	-60.0	-10.00	13.3	
	Week 20	Peg	6	5 (83.3)	2.67 (10.11)	-6.7	0.00	20.0	0.68 (0.83) [-0.71, 2.59]
		SoC	5	4 (80.0)	-15.00 (33.28)	-60.0	-6.67	13.3	
	Week 26	Peg	6	4 (66.7)	1.67 (8.39)	-6.7	0.00	13.3	-0.87 (1.35) [-4.17, 1.00]
		SoC	5	2 (40.0)	10.00 (4.71)	6.7	10.00	13.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SICD: Change from baseline in QLQ-C30 Physical Functioning (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.9309

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SICD: Change from baseline in QLQ-C30 Physical Functioning (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Male	Peg	Week 4	19	16 (84.2)	6.77 (2.70)	(1.13, 12.42)	13.17 (4.67)	(3.44, 22.91)	0.0105	*
		Week 8	19	15 (78.9)	6.49 (3.04)	(0.14, 12.85)	12.86 (5.17)	(2.05, 23.67)	0.0222	*
		Week 12	19	17 (89.5)	5.85 (2.86)	(-0.09, 11.79)	11.16 (5.07)	(0.62, 21.70)	0.0390	*
		Week 16	19	16 (84.2)	9.05 (3.46)	(1.86, 16.24)	12.77 (5.85)	(0.60, 24.94)	0.0406	*
		Week 20	19	17 (89.5)	6.92 (3.14)	(0.38, 13.46)	9.73 (5.59)	(-1.89, 21.35)	0.0963	
		Week 26	19	14 (73.7)	6.91 (4.25)	(-2.10, 15.92)	9.70 (7.78)	(-6.79, 26.20)	0.2302	
	SoC	Week 4	10	8 (80.0)	-6.40 (3.84)	(-14.42, 1.62)				
		Week 8	10	8 (80.0)	-6.37 (4.20)	(-15.15, 2.42)				
		Week 12	10	8 (80.0)	-5.31 (4.20)	(-14.05, 3.43)				
		Week 16	10	9 (90.0)	-3.72 (4.70)	(-13.48, 6.05)				
		Week 20	10	8 (80.0)	-2.81 (4.64)	(-12.45, 6.84)				
		Week 26	10	6 (60.0)	-2.79 (6.50)	(-16.57, 10.99)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SICD: Change from baseline in QLQ-C30 Physical Functioning (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Female	Peg	Week 4	16	15 (93.8)	9.21 (4.73)	(-0.72, 19.14)	18.82 (10.10)	(-2.39, 40.04)	0.0787	
		Week 8	16	14 (87.5)	10.95 (4.73)	(0.97, 20.92)	13.31 (10.25)	(-8.31, 34.93)	0.2112	
		Week 12	16	14 (87.5)	12.04 (4.84)	(1.82, 22.25)	13.91 (10.49)	(-8.23, 36.04)	0.2026	
		Week 16	16	14 (87.5)	12.44 (5.13)	(1.62, 23.27)	17.47 (11.12)	(-5.99, 40.93)	0.1346	
		Week 20	16	15 (93.8)	12.77 (4.37)	(3.59, 21.95)	20.40 (9.33)	(0.79, 40.01)	0.0423	*
		Week 26	16	13 (81.3)	12.95 (2.72)	(7.15, 18.75)	8.90 (5.99)	(-3.88, 21.67)	0.1585	
	SoC	Week 4	8	7 (87.5)	-9.61 (8.34)	(-27.15, 7.92)				
		Week 8	8	7 (87.5)	-2.37 (8.16)	(-19.59, 14.85)				
		Week 12	8	7 (87.5)	-1.87 (8.36)	(-19.50, 15.77)				
		Week 16	8	7 (87.5)	-5.03 (8.86)	(-23.72, 13.66)				
		Week 20	8	7 (87.5)	-7.64 (7.71)	(-23.84, 8.57)				
		Week 26	8	6 (75.0)	4.05 (4.89)	(-6.37, 14.47)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SIHD: Change from baseline in QLQ-C30 Physical Functioning (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Physical Functioning (censored)	Male	Baseline	Peg	19	19 (100.0)	81.05 (14.78)	46.7	86.67	100.0	
			SoC	10	9 (90.0)	74.81 (21.55)	26.7	80.00	100.0	
		Week 4	Peg	19	16 (84.2)	88.75 (12.10)	53.3	93.33	100.0	
			SoC	10	9 (90.0)	77.78 (14.53)	60.0	80.00	100.0	
		Week 8	Peg	19	15 (78.9)	88.89 (13.49)	53.3	93.33	100.0	
			SoC	10	9 (90.0)	77.78 (14.91)	60.0	80.00	100.0	
		Week 12	Peg	19	17 (89.5)	88.24 (13.44)	60.0	93.33	100.0	
			SoC	10	9 (90.0)	78.52 (14.44)	53.3	80.00	100.0	
		Week 16	Peg	19	16 (84.2)	88.75 (17.68)	33.3	96.67	100.0	
			SoC	10	10 (100.0)	76.00 (17.55)	40.0	80.00	100.0	
		Week 20	Peg	19	17 (89.5)	86.67 (16.67)	46.7	86.67	100.0	
			SoC	10	9 (90.0)	80.00 (11.55)	60.0	80.00	100.0	
		Week 26	Peg	19	14 (73.7)	86.67 (18.49)	33.3	90.00	100.0	
			SoC	10	7 (70.0)	79.05 (13.57)	53.3	80.00	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SIHD: Change from baseline in QLQ-C30 Physical Functioning (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Physical Functioning (censored)	Female	Baseline	Peg	16	16 (100.0)	79.58 (17.80)	40.0	86.67	100.0	
			SoC	8	7 (87.5)	84.76 (9.97)	66.7	86.67	93.3	
		Week 4	Peg	16	15 (93.8)	92.00 (8.43)	73.3	93.33	100.0	
			SoC	8	8 (100.0)	76.67 (30.65)	6.7	86.67	100.0	
		Week 8	Peg	16	14 (87.5)	93.81 (6.11)	86.7	93.33	100.0	
			SoC	8	7 (87.5)	77.14 (27.98)	20.0	93.33	100.0	
		Week 12	Peg	16	14 (87.5)	94.76 (5.95)	86.7	96.67	100.0	
			SoC	8	7 (87.5)	78.10 (28.73)	20.0	93.33	100.0	
		Week 16	Peg	16	14 (87.5)	94.29 (7.78)	73.3	96.67	100.0	
			SoC	8	7 (87.5)	78.10 (28.73)	20.0	93.33	100.0	
		Week 20	Peg	16	15 (93.8)	95.56 (6.00)	80.0	100.00	100.0	
			SoC	8	8 (100.0)	79.17 (26.77)	20.0	90.00	100.0	
		Week 26	Peg	16	13 (81.3)	94.87 (5.55)	86.7	93.33	100.0	
			SoC	8	6 (75.0)	86.67 (13.98)	66.7	90.00	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SIHD: Change from baseline in QLQ-C30 Physical Functioning (censored) by sex
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Male in QLQ-C30 Physical Functioning (censored)	Week 4	Peg	19	16 (84.2)	6.67 (13.11)	-20.0	10.00	26.7	1.01 (0.48) [0.16, 2.07]
		SoC	10	8 (80.0)	-5.83 (9.04)	-20.0	-3.33	6.7	
	Week 8	Peg	19	15 (78.9)	6.22 (13.44)	-20.0	6.67	26.7	0.93 (0.49) [0.07, 1.99]
		SoC	10	8 (80.0)	-5.83 (10.35)	-20.0	-6.67	6.7	
	Week 12	Peg	19	17 (89.5)	5.49 (13.99)	-13.3	6.67	33.3	0.80 (0.47) [-0.04, 1.80]
		SoC	10	8 (80.0)	-5.00 (9.26)	-20.0	-6.67	6.7	
	Week 16	Peg	19	16 (84.2)	8.33 (16.24)	-40.0	10.00	26.7	0.62 (0.45) [-0.20, 1.57]
		SoC	10	9 (90.0)	-1.48 (13.24)	-26.7	0.00	13.3	
	Week 20	Peg	19	17 (89.5)	7.06 (14.62)	-20.0	6.67	26.7	0.73 (0.46) [-0.12, 1.71]
		SoC	10	8 (80.0)	-3.33 (11.82)	-20.0	-3.33	13.3	
	Week 26	Peg	19	14 (73.7)	6.67 (18.12)	-40.0	10.00	26.7	0.53 (0.53) [-0.44, 1.65]
		SoC	10	6 (60.0)	-2.22 (8.07)	-13.3	-3.33	6.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SIHD: Change from baseline in QLQ-C30 Physical Functioning (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Physical Functioning (censored)	Female	Week 4	Peg	16	15 (93.8)	9.78 (10.35)	-6.7	6.67	26.7	1.14 (0.52) [0.24, 2.30]
			SoC	8	7 (87.5)	-11.43 (28.73)	-73.3	0.00	6.7	
		Week 8	Peg	16	14 (87.5)	12.86 (14.19)	0.0	10.00	53.3	1.06 (0.53) [0.16, 2.23]
			SoC	8	7 (87.5)	-7.62 (25.37)	-60.0	0.00	13.3	
		Week 12	Peg	16	14 (87.5)	13.81 (13.95)	0.0	13.33	53.3	1.05 (0.53) [0.15, 2.22]
			SoC	8	7 (87.5)	-6.67 (26.11)	-60.0	0.00	13.3	
		Week 16	Peg	16	14 (87.5)	13.33 (16.74)	-13.3	13.33	53.3	0.96 (0.52) [0.05, 2.10]
			SoC	8	7 (87.5)	-6.67 (25.82)	-60.0	6.67	13.3	
		Week 20	Peg	16	15 (93.8)	13.33 (16.13)	-6.7	13.33	53.3	1.09 (0.52) [0.20, 2.24]
			SoC	8	7 (87.5)	-8.57 (25.16)	-60.0	0.00	13.3	
		Week 26	Peg	16	13 (81.3)	14.36 (16.52)	-6.7	13.33	53.3	0.81 (0.55) [-0.16, 2.01]
			SoC	8	6 (75.0)	1.11 (12.94)	-20.0	3.33	13.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SICB: Change from baseline in QLQ-C30 Physical Functioning (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.6005

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SICB: Change from baseline in QLQ-C30 Physical Functioning (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Latin America	Peg	Week 4	12	11 (91.7)	10.36 (1.08)	(7.93, 12.80)	14.21 (2.74)	(8.02, 20.40)	0.0006	*
		Week 8	12	10 (83.3)	10.27 (2.43)	(4.66, 15.88)	14.96 (5.97)	(1.18, 28.73)	0.0367	*
		Week 12	12	11 (91.7)	10.46 (2.27)	(5.33, 15.58)	14.38 (5.76)	(1.34, 27.42)	0.0342	*
		Week 16	12	10 (83.3)	13.62 (1.22)	(10.82, 16.43)	18.42 (2.99)	(11.52, 25.31)	0.0003	*
		Week 20	12	10 (83.3)	13.33 (1.84)	(9.08, 17.58)	16.67 (4.52)	(6.25, 27.08)	0.0061	*
		Week 26	12	9 (75.0)	12.61 (3.51)	(4.30, 20.91)	15.05 (8.22)	(-4.37, 34.48)	0.1096	
	SoC	Week 4	2	2 (100.0)	-3.84 (2.52)	(-9.53, 1.85)				
		Week 8	2	2 (100.0)	-4.69 (5.45)	(-17.25, 7.88)				
		Week 12	2	2 (100.0)	-3.92 (5.30)	(-15.91, 8.06)				
		Week 16	2	2 (100.0)	-4.79 (2.73)	(-11.08, 1.50)				
		Week 20	2	2 (100.0)	-3.33 (4.12)	(-12.84, 6.17)				
		Week 26	2	2 (100.0)	-2.45 (7.42)	(-19.98, 15.09)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SICB: Change from baseline in QLQ-C30 Physical Functioning (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Asia Pacific Peg		Week 4	23	20 (87.0)	6.40 (3.69)	(-1.15, 13.95)	14.57 (6.06)	(2.18, 26.97)	0.0228	*
		Week 8	23	19 (82.6)	8.15 (3.72)	(0.53, 15.78)	13.92 (6.04)	(1.54, 26.29)	0.0288	*
		Week 12	23	20 (87.0)	8.17 (3.71)	(0.58, 15.77)	13.53 (6.10)	(1.05, 26.00)	0.0345	*
		Week 16	23	20 (87.0)	8.92 (4.02)	(0.71, 17.13)	12.14 (6.38)	(-0.89, 25.17)	0.0668	
		Week 20	23	22 (95.7)	7.90 (3.59)	(0.57, 15.22)	13.06 (6.07)	(0.69, 25.43)	0.0392	*
		Week 26	23	18 (78.3)	8.80 (3.60)	(1.38, 16.22)	7.78 (6.38)	(-5.39, 20.94)	0.2345	
SoC		Week 4	16	13 (81.3)	-8.17 (4.66)	(-17.71, 1.36)				
		Week 8	16	13 (81.3)	-5.76 (4.55)	(-15.08, 3.56)				
		Week 12	16	13 (81.3)	-5.36 (4.69)	(-14.95, 4.24)				
		Week 16	16	14 (87.5)	-3.22 (4.83)	(-13.09, 6.65)				
		Week 20	16	13 (81.3)	-5.17 (4.76)	(-14.87, 4.54)				
		Week 26	16	10 (62.5)	1.02 (5.15)	(-9.60, 11.64)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SIHb: Change from baseline in QLQ-C30 Physical Functioning (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Physical Functioning (censored)	Latin America	Baseline	Peg	12	12 (100.0)	80.56 (17.86)	40.0	83.33	100.0	
			SoC	2	2 (100.0)	83.33 (14.14)	73.3	83.33	93.3	
		Week 4	Peg	12	11 (91.7)	94.55 (5.83)	86.7	93.33	100.0	
			SoC	2	2 (100.0)	80.00 (0.00)	80.0	80.00	80.0	
		Week 8	Peg	12	10 (83.3)	95.33 (7.73)	80.0	100.00	100.0	
			SoC	2	2 (100.0)	80.00 (0.00)	80.0	80.00	80.0	
		Week 12	Peg	12	11 (91.7)	94.55 (7.78)	80.0	100.00	100.0	
			SoC	2	2 (100.0)	80.00 (0.00)	80.0	80.00	80.0	
		Week 16	Peg	12	10 (83.3)	98.67 (4.22)	86.7	100.00	100.0	
			SoC	2	2 (100.0)	80.00 (0.00)	80.0	80.00	80.0	
		Week 20	Peg	12	10 (83.3)	96.67 (6.48)	80.0	100.00	100.0	
			SoC	2	2 (100.0)	80.00 (0.00)	80.0	80.00	80.0	
		Week 26	Peg	12	9 (75.0)	94.07 (11.28)	66.7	100.00	100.0	
			SoC	2	2 (100.0)	80.00 (0.00)	80.0	80.00	80.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SIHB: Change from baseline in QLQ-C30 Physical Functioning (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Physical Functioning (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	80.29 (15.37)	46.7	86.67	100.0	
			SoC	16	14 (87.5)	78.57 (18.52)	26.7	80.00	100.0	
		Week 4	Peg	23	20 (87.0)	88.00 (11.77)	53.3	93.33	100.0	
			SoC	16	15 (93.8)	76.89 (24.28)	6.7	80.00	100.0	
		Week 8	Peg	23	19 (82.6)	89.12 (11.59)	53.3	93.33	100.0	
			SoC	16	14 (87.5)	77.14 (22.30)	20.0	76.67	100.0	
		Week 12	Peg	23	20 (87.0)	89.33 (12.31)	60.0	93.33	100.0	
			SoC	16	14 (87.5)	78.10 (22.56)	20.0	80.00	100.0	
		Week 16	Peg	23	20 (87.0)	87.67 (15.79)	33.3	93.33	100.0	
			SoC	16	15 (93.8)	76.44 (23.48)	20.0	80.00	100.0	
		Week 20	Peg	23	22 (95.7)	88.18 (14.97)	46.7	93.33	100.0	
			SoC	16	15 (93.8)	79.56 (20.85)	20.0	80.00	100.0	
		Week 26	Peg	23	18 (78.3)	88.89 (15.51)	33.3	93.33	100.0	
			SoC	16	11 (68.8)	83.03 (15.02)	53.3	80.00	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SIHB: Change from baseline in QLQ-C30 Physical Functioning (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Physical Functioning (censored)	Latin America	Week 4	Peg	12	11 (91.7)	10.30 (8.62)	0.0	13.33	20.0	1.37 (0.92) [-0.14, 3.50]
			SoC	2	2 (100.0)	-3.33 (14.14)	-13.3	-3.33	6.7	
		Week 8	Peg	12	10 (83.3)	10.00 (12.67)	-13.3	13.33	26.7	0.96 (0.91) [-0.59, 3.00]
			SoC	2	2 (100.0)	-3.33 (14.14)	-13.3	-3.33	6.7	
		Week 12	Peg	12	11 (91.7)	10.30 (12.06)	-13.3	13.33	26.7	1.03 (0.89) [-0.50, 3.03]
			SoC	2	2 (100.0)	-3.33 (14.14)	-13.3	-3.33	6.7	
		Week 16	Peg	12	10 (83.3)	13.33 (11.76)	0.0	13.33	33.3	1.28 (0.94) [-0.25, 3.45]
			SoC	2	2 (100.0)	-3.33 (14.14)	-13.3	-3.33	6.7	
		Week 20	Peg	12	10 (83.3)	13.33 (11.33)	0.0	13.33	26.7	1.32 (0.94) [-0.20, 3.51]
			SoC	2	2 (100.0)	-3.33 (14.14)	-13.3	-3.33	6.7	
		Week 26	Peg	12	9 (75.0)	12.59 (12.22)	-6.7	13.33	26.7	1.17 (0.95) [-0.38, 3.39]
			SoC	2	2 (100.0)	-3.33 (14.14)	-13.3	-3.33	6.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SIHB: Change from baseline in QLQ-C30 Physical Functioning (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Physical Functioning (censored)	Asia	Week 4	Peg	23	20 (87.0)	7.00 (13.24)	-20.0	6.67	26.7	0.94 (0.39) [0.25, 1.78]
			SoC	16	13 (81.3)	-9.23 (21.18)	-73.3	0.00	6.7	
	Pacific	Week 8	Peg	23	19 (82.6)	9.12 (14.94)	-20.0	6.67	53.3	0.95 (0.39) [0.24, 1.80]
			SoC	16	13 (81.3)	-7.18 (19.14)	-60.0	0.00	13.3	
		Week 12	Peg	23	20 (87.0)	8.67 (15.76)	-13.3	6.67	53.3	0.84 (0.39) [0.14, 1.66]
			SoC	16	13 (81.3)	-6.15 (19.33)	-60.0	0.00	13.3	
		Week 16	Peg	23	20 (87.0)	9.33 (18.40)	-40.0	10.00	53.3	0.67 (0.37) [-0.01, 1.45]
			SoC	16	14 (87.5)	-3.81 (20.21)	-60.0	3.33	13.3	
		Week 20	Peg	23	22 (95.7)	8.48 (16.99)	-20.0	6.67	53.3	0.79 (0.38) [0.11, 1.59]
			SoC	16	13 (81.3)	-6.15 (19.71)	-60.0	0.00	13.3	
		Week 26	Peg	23	18 (78.3)	9.26 (19.82)	-40.0	6.67	53.3	0.52 (0.42) [-0.25, 1.39]
			SoC	16	10 (62.5)	0.00 (10.42)	-20.0	0.00	13.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SICA: Change from baseline in QLQ-C30 Physical Functioning (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.2422

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SICA: Change from baseline in QLQ-C30 Physical Functioning (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 4	21	18 (85.7)	9.16 (1.79)	(5.42, 12.89)	15.45 (3.85)	(7.42, 23.48)	0.0007	*
		Week 8	21	17 (81.0)	10.30 (2.53)	(5.00, 15.60)	17.32 (5.33)	(6.17, 28.47)	0.0042	*
		Week 12	21	18 (85.7)	10.03 (2.80)	(4.18, 15.88)	18.12 (6.03)	(5.55, 30.70)	0.0070	*
		Week 16	21	17 (81.0)	13.96 (2.56)	(8.62, 19.31)	19.09 (5.11)	(8.43, 29.74)	0.0013	*
		Week 20	21	18 (85.7)	9.95 (3.07)	(3.56, 16.34)	13.77 (6.57)	(0.06, 27.48)	0.0492	*
	Week 26	21	15 (71.4)	12.11 (2.73)	(6.29, 17.92)	19.32 (6.79)	(4.85, 33.79)	0.0123	*	
	SoC	Week 4	8	5 (62.5)	-6.29 (3.40)	(-13.39, 0.81)				
		Week 8	8	5 (62.5)	-7.02 (4.68)	(-16.81, 2.77)				
		Week 12	8	5 (62.5)	-8.10 (5.33)	(-19.21, 3.02)				
		Week 16	8	6 (75.0)	-5.12 (4.37)	(-14.23, 3.98)				
		Week 20	8	5 (62.5)	-3.82 (5.82)	(-15.95, 8.31)				
Week 26	8	3 (37.5)	-7.21 (6.18)	(-20.38, 5.96)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SICA: Change from baseline in QLQ-C30 Physical Functioning (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	6.71 (5.20)	(-4.14, 17.57)	16.11 (7.92)	(-0.41, 32.63)	0.0554	
		Week 8	14	12 (85.7)	8.24 (4.97)	(-2.16, 18.64)	14.80 (7.44)	(-0.78, 30.37)	0.0614	
		Week 12	14	13 (92.9)	8.23 (4.59)	(-1.34, 17.81)	12.93 (7.00)	(-1.66, 27.53)	0.0794	
		Week 16	14	13 (92.9)	7.75 (5.52)	(-3.76, 19.25)	12.48 (8.41)	(-5.07, 30.03)	0.1535	
		Week 20	14	14 (100.0)	9.64 (4.48)	(0.32, 18.95)	15.79 (6.95)	(1.35, 30.24)	0.0336	*
		Week 26	14	12 (85.7)	8.28 (4.60)	(-1.39, 17.95)	6.73 (7.15)	(-8.29, 21.74)	0.3591	
	SoC	Week 4	10	10 (100.0)	-9.40 (5.94)	(-21.78, 2.99)				
		Week 8	10	10 (100.0)	-6.56 (5.45)	(-17.97, 4.86)				
		Week 12	10	10 (100.0)	-4.70 (5.24)	(-15.63, 6.23)				
		Week 16	10	10 (100.0)	-4.74 (6.30)	(-17.87, 8.40)				
		Week 20	10	10 (100.0)	-6.16 (5.30)	(-17.18, 4.87)				
		Week 26	10	9 (90.0)	1.55 (5.34)	(-9.66, 12.77)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SIHA: Change from baseline in QLQ-C30 Physical Functioning (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Physical Functioning (censored)	< 4	Baseline	Peg	21	21 (100.0)	79.05 (18.05)	40.0	86.67	100.0	
			SoC	8	6 (75.0)	71.11 (25.53)	26.7	76.67	100.0	
		Week 4	Peg	21	18 (85.7)	91.48 (9.09)	73.3	93.33	100.0	
			SoC	8	7 (87.5)	81.90 (17.94)	60.0	80.00	100.0	
		Week 8	Peg	21	17 (81.0)	92.94 (9.27)	73.3	100.00	100.0	
			SoC	8	6 (75.0)	78.89 (18.09)	60.0	76.67	100.0	
		Week 12	Peg	21	18 (85.7)	92.22 (11.26)	60.0	100.00	100.0	
			SoC	8	6 (75.0)	77.78 (18.22)	53.3	80.00	100.0	
		Week 16	Peg	21	17 (81.0)	92.94 (10.13)	73.3	100.00	100.0	
			SoC	8	7 (87.5)	74.29 (21.23)	40.0	80.00	100.0	
		Week 20	Peg	21	18 (85.7)	89.63 (14.50)	46.7	96.67	100.0	
			SoC	8	7 (87.5)	82.86 (15.33)	60.0	80.00	100.0	
		Week 26	Peg	21	15 (71.4)	92.00 (9.82)	66.7	93.33	100.0	
			SoC	8	4 (50.0)	78.33 (19.15)	53.3	80.00	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SIHA: Change from baseline in QLQ-C30 Physical Functioning (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Physical Functioning (censored)	>= 4	Baseline	Peg	14	14 (100.0)	82.38 (12.70)	66.7	83.33	100.0	
			SoC	10	10 (100.0)	84.00 (9.53)	66.7	83.33	93.3	
	Week 4	Peg	14	13 (92.9)	88.72 (12.29)	53.3	93.33	100.0		
		SoC	10	10 (100.0)	74.00 (26.00)	6.7	80.00	100.0		
	Week 8	Peg	14	12 (85.7)	88.89 (12.50)	53.3	90.00	100.0		
		SoC	10	10 (100.0)	76.67 (23.15)	20.0	80.00	100.0		
	Week 12	Peg	14	13 (92.9)	89.74 (11.09)	60.0	93.33	100.0		
		SoC	10	10 (100.0)	78.67 (23.48)	20.0	80.00	100.0		
	Week 16	Peg	14	13 (92.9)	89.23 (18.16)	33.3	93.33	100.0		
		SoC	10	10 (100.0)	78.67 (23.48)	20.0	80.00	100.0		
	Week 20	Peg	14	14 (100.0)	92.38 (12.22)	53.3	93.33	100.0		
		SoC	10	10 (100.0)	77.33 (22.49)	20.0	80.00	100.0		
	Week 26	Peg	14	12 (85.7)	88.89 (18.71)	33.3	93.33	100.0		
		SoC	10	9 (90.0)	84.44 (11.55)	66.7	80.00	100.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SIHA: Change from baseline in QLQ-C30 Physical Functioning (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in QLQ-C30 Physical Functioning (censored)	Week 4	Peg	21	18 (85.7)	8.89 (9.97)	-6.7	6.67	26.7	1.35 (0.58) [0.36, 2.64]
		SoC	8	5 (62.5)	-5.33 (10.95)	-20.0	0.00	6.7	
	Week 8	Peg	21	17 (81.0)	9.80 (15.30)	-13.3	6.67	53.3	1.00 (0.56) [0.01, 2.23]
		SoC	8	5 (62.5)	-5.33 (10.95)	-20.0	0.00	6.7	
	Week 12	Peg	21	18 (85.7)	9.63 (16.68)	-13.3	10.00	53.3	1.01 (0.56) [0.02, 2.22]
		SoC	8	5 (62.5)	-6.67 (9.43)	-20.0	-6.67	6.7	
	Week 16	Peg	21	17 (81.0)	12.55 (15.25)	-13.3	13.33	53.3	0.86 (0.52) [-0.07, 1.98]
		SoC	8	6 (75.0)	-1.11 (15.44)	-26.7	-0.00	13.3	
	Week 20	Peg	21	18 (85.7)	10.00 (16.69)	-20.0	6.67	53.3	0.83 (0.55) [-0.16, 2.01]
		SoC	8	5 (62.5)	-4.00 (13.82)	-20.0	-6.67	13.3	
	Week 26	Peg	21	15 (71.4)	11.11 (16.65)	-6.7	6.67	53.3	0.80 (0.70) [-0.45, 2.30]
		SoC	8	3 (37.5)	-2.22 (7.70)	-6.7	-6.67	6.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QYWC_SIHA: Change from baseline in QLQ-C30 Physical Functioning (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline \geq 4 in QLQ-C30 Physical Functioning (censored)	Week 4	Peg	14	13 (92.9)	7.18 (14.26)	-20.0	13.33	26.7	0.87 (0.47) [0.05, 1.89]
		SoC	10	10 (100.0)	-10.00 (23.78)	-73.3	0.00	6.7	
	Week 8	Peg	14	12 (85.7)	8.89 (12.50)	-20.0	10.00	20.0	0.91 (0.48) [0.07, 1.96]
		SoC	10	10 (100.0)	-7.33 (21.42)	-60.0	0.00	13.3	
	Week 12	Peg	14	13 (92.9)	8.72 (11.02)	-13.3	13.33	20.0	0.82 (0.46) [-0.01, 1.82]
		SoC	10	10 (100.0)	-5.33 (21.95)	-60.0	0.00	13.3	
	Week 16	Peg	14	13 (92.9)	8.21 (18.08)	-40.0	13.33	33.3	0.66 (0.46) [-0.17, 1.64]
		SoC	10	10 (100.0)	-5.33 (21.73)	-60.0	3.33	13.3	
	Week 20	Peg	14	14 (100.0)	10.00 (14.26)	-20.0	10.00	26.7	0.92 (0.46) [0.11, 1.92]
		SoC	10	10 (100.0)	-6.67 (21.31)	-60.0	0.00	13.3	
	Week 26	Peg	14	12 (85.7)	9.44 (19.17)	-40.0	13.33	33.3	0.55 (0.48) [-0.32, 1.56]
		SoC	10	9 (90.0)	0.00 (11.55)	-20.0	0.00	13.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SICC: Change from baseline in QLQ-C30 Role Functioning (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.1925

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SICC: Change from baseline in QLQ-C30 Role Functioning (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 60	Peg	Week 4	29	26 (89.7)	14.15 (2.97)	(8.10, 20.21)	21.62 (5.60)	(10.22, 33.02)	0.0005	*
		Week 8	29	24 (82.8)	10.64 (3.56)	(3.37, 17.91)	17.36 (6.50)	(4.10, 30.62)	0.0120	*
		Week 12	29	26 (89.7)	12.50 (3.14)	(6.12, 18.87)	17.72 (5.88)	(5.76, 29.68)	0.0049	*
		Week 16	29	25 (86.2)	10.03 (4.03)	(1.83, 18.22)	18.50 (7.10)	(4.04, 32.95)	0.0137	*
		Week 20	29	27 (93.1)	9.54 (3.01)	(3.42, 15.66)	13.25 (5.74)	(1.58, 24.92)	0.0272	*
	Week 26	29	23 (79.3)	12.99 (3.13)	(6.59, 19.39)	19.67 (5.84)	(7.73, 31.62)	0.0021	*	
	SoC	Week 4	13	11 (84.6)	-7.46 (4.63)	(-16.89, 1.97)				
		Week 8	13	11 (84.6)	-6.72 (5.31)	(-17.56, 4.11)				
		Week 12	13	11 (84.6)	-5.23 (4.87)	(-15.14, 4.68)				
		Week 16	13	12 (92.3)	-8.47 (5.81)	(-20.30, 3.36)				
		Week 20	13	11 (84.6)	-3.71 (4.79)	(-13.45, 6.02)				
	Week 26	13	10 (76.9)	-6.68 (4.84)	(-16.58, 3.21)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SICC: Change from baseline in QLQ-C30 Role Functioning (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	1.56 (14.72)	(-36.27, 39.39)	25.69 (23.27)	(-34.13, 85.50)	0.3199	
		Week 8	6	5 (83.3)	9.55 (10.90)	(-18.48, 37.57)	15.98 (17.24)	(-28.33, 60.29)	0.3964	
		Week 12	6	5 (83.3)	2.89 (13.34)	(-31.41, 37.18)	18.15 (21.09)	(-36.08, 72.37)	0.4290	
		Week 16	6	5 (83.3)	-2.97 (15.11)	(-41.81, 35.87)	9.15 (24.04)	(-52.65, 70.95)	0.7191	
		Week 20	6	5 (83.3)	9.30 (13.81)	(-26.20, 44.80)	25.82 (21.97)	(-30.66, 82.29)	0.2929	
		Week 26	6	4 (66.7)	10.13 (4.20)	(-7.96, 28.22)	-7.84 (8.20)	(-43.14, 27.45)	0.4399	
	SoC	Week 4	5	4 (80.0)	-24.12 (17.20)	(-68.34, 20.09)				
		Week 8	5	4 (80.0)	-6.43 (12.74)	(-39.19, 26.32)				
		Week 12	5	4 (80.0)	-15.26 (15.59)	(-55.34, 24.82)				
		Week 16	5	4 (80.0)	-12.12 (17.44)	(-56.94, 32.70)				
		Week 20	5	4 (80.0)	-16.52 (15.93)	(-57.48, 24.44)				
		Week 26	5	2 (40.0)	17.97 (7.01)	(-12.17, 48.11)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SIHC: Change from baseline in QLQ-C30 Role Functioning (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Role Functioning (censored)	< 60	Baseline	Peg	29	29 (100.0)	70.11 (29.67)	0.0	66.67	100.0	
			SoC	13	12 (92.3)	79.17 (29.41)	0.0	83.33	100.0	
		Week 4	Peg	29	26 (89.7)	90.38 (13.48)	66.7	100.00	100.0	
			SoC	13	12 (92.3)	73.61 (19.41)	33.3	66.67	100.0	
		Week 8	Peg	29	24 (82.8)	89.58 (16.89)	33.3	100.00	100.0	
			SoC	13	11 (84.6)	72.73 (17.12)	50.0	66.67	100.0	
		Week 12	Peg	29	26 (89.7)	89.10 (16.29)	50.0	100.00	100.0	
			SoC	13	11 (84.6)	75.76 (20.23)	33.3	66.67	100.0	
		Week 16	Peg	29	25 (86.2)	87.33 (19.41)	33.3	100.00	100.0	
			SoC	13	12 (92.3)	69.44 (25.46)	0.0	66.67	100.0	
		Week 20	Peg	29	27 (93.1)	85.19 (18.10)	33.3	83.33	100.0	
			SoC	13	12 (92.3)	77.78 (14.79)	66.7	66.67	100.0	
		Week 26	Peg	29	23 (79.3)	89.86 (17.22)	50.0	100.00	100.0	
			SoC	13	10 (76.9)	73.33 (11.65)	66.7	66.67	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SIHC: Change from baseline in QLQ-C30 Role Functioning (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Role Functioning (censored)	>= 60	Baseline	Peg	6	6 (100.0)	77.78 (29.19)	33.3	91.67	100.0	
			SoC	5	4 (80.0)	91.67 (9.62)	83.3	91.67	100.0	
	Week 4	Peg	6	5 (83.3)	90.00 (14.91)	66.7	100.00	100.0		
		SoC	5	5 (100.0)	66.67 (40.82)	0.0	66.67	100.0		
	Week 8	Peg	6	5 (83.3)	96.67 (7.45)	83.3	100.00	100.0		
		SoC	5	5 (100.0)	83.33 (28.87)	33.3	100.00	100.0		
	Week 12	Peg	6	5 (83.3)	90.00 (14.91)	66.7	100.00	100.0		
		SoC	5	5 (100.0)	76.67 (32.49)	33.3	100.00	100.0		
	Week 16	Peg	6	5 (83.3)	76.67 (22.36)	50.0	66.67	100.0		
		SoC	5	5 (100.0)	76.67 (32.49)	33.3	100.00	100.0		
	Week 20	Peg	6	5 (83.3)	86.67 (21.73)	50.0	100.00	100.0		
		SoC	5	5 (100.0)	76.67 (32.49)	33.3	100.00	100.0		
	Week 26	Peg	6	4 (66.7)	95.83 (8.33)	83.3	100.00	100.0		
		SoC	5	3 (60.0)	100.00 (0.00)	100.0	100.00	100.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SIHC: Change from baseline in QLQ-C30 Role Functioning (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in QLQ-C30 Role Functioning (censored)	Week 4	Peg	29	26 (89.7)	17.31 (28.47)	-33.3	16.67	83.3	1.25 (0.40) [0.53, 2.12]
		SoC	13	11 (84.6)	-15.15 (15.73)	-33.3	-16.67	16.7	
	Week 8	Peg	29	24 (82.8)	13.89 (27.22)	-66.7	16.67	66.7	1.03 (0.40) [0.31, 1.89]
		SoC	13	11 (84.6)	-13.64 (23.35)	-50.0	-16.67	16.7	
	Week 12	Peg	29	26 (89.7)	14.74 (23.72)	-33.3	8.33	66.7	1.13 (0.40) [0.42, 1.98]
		SoC	13	11 (84.6)	-10.61 (17.12)	-33.3	-16.67	16.7	
	Week 16	Peg	29	25 (86.2)	10.67 (28.41)	-66.7	16.67	66.7	0.77 (0.37) [0.08, 1.56]
		SoC	13	12 (92.3)	-9.72 (19.41)	-33.3	-16.67	16.7	
	Week 20	Peg	29	27 (93.1)	12.35 (23.84)	-50.0	16.67	66.7	0.98 (0.39) [0.28, 1.81]
		SoC	13	11 (84.6)	-10.61 (20.10)	-33.3	-16.67	16.7	
	Week 26	Peg	29	23 (79.3)	15.22 (25.08)	-50.0	16.67	66.7	1.10 (0.42) [0.35, 2.00]
		SoC	13	10 (76.9)	-11.67 (20.86)	-33.3	-16.67	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SIHC: Change from baseline in QLQ-C30 Role Functioning (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline >= 60 in QLQ-C30 Role Functioning (censored)	Week 4	Peg	6	5 (83.3)	6.67 (36.51)	-33.3	0.00	66.7	0.98	(0.87) [-0.36, 3.07]
		SoC	5	4 (80.0)	-33.33 (36.00)	-83.3	-25.00	0.0		
	Week 8	Peg	6	5 (83.3)	13.33 (29.81)	0.0	0.00	66.7	0.82	(0.85) [-0.54, 2.82]
		SoC	5	4 (80.0)	-12.50 (25.00)	-50.0	0.00	0.0		
	Week 12	Peg	6	5 (83.3)	6.67 (36.51)	-33.3	0.00	66.7	0.76	(0.84) [-0.61, 2.72]
		SoC	5	4 (80.0)	-20.83 (25.00)	-50.0	-16.67	0.0		
	Week 16	Peg	6	5 (83.3)	3.33 (38.01)	-33.3	0.00	66.7	0.65	(0.83) [-0.74, 2.55]
		SoC	5	4 (80.0)	-20.83 (25.00)	-50.0	-16.67	0.0		
	Week 20	Peg	6	5 (83.3)	13.33 (29.81)	0.0	0.00	66.7	1.09	(0.89) [-0.24, 3.26]
		SoC	5	4 (80.0)	-20.83 (25.00)	-50.0	-16.67	0.0		
	Week 26	Peg	6	4 (66.7)	16.67 (33.33)	0.0	0.00	66.7	0.46	(1.26) [-1.62, 3.30]
		SoC	5	2 (40.0)	0.00 (0.00)	0.0	0.00	0.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SICD: Change from baseline in QLQ-C30 Role Functioning (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.3249

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SICD: Change from baseline in QLQ-C30 Role Functioning (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Male	Peg	Week 4	19	16 (84.2)	9.12 (4.30)	(0.15, 18.09)	15.26 (7.73)	(-0.86, 31.38)	0.0622	
		Week 8	19	15 (78.9)	5.57 (4.94)	(-4.77, 15.92)	15.23 (8.64)	(-2.85, 33.30)	0.0940	
		Week 12	19	17 (89.5)	4.91 (4.02)	(-3.45, 13.28)	18.10 (7.39)	(2.73, 33.48)	0.0232	*
		Week 16	19	16 (84.2)	3.02 (5.19)	(-7.77, 13.81)	15.26 (8.82)	(-3.09, 33.61)	0.0983	
		Week 20	19	17 (89.5)	6.21 (4.18)	(-2.49, 14.91)	13.09 (7.97)	(-3.48, 29.65)	0.1154	
		Week 26	19	14 (73.7)	6.49 (4.32)	(-2.66, 15.65)	13.31 (8.02)	(-3.69, 30.31)	0.1165	
	SoC	Week 4	10	8 (80.0)	-6.14 (6.36)	(-19.40, 7.13)				
		Week 8	10	8 (80.0)	-9.65 (7.01)	(-24.32, 5.01)				
		Week 12	10	8 (80.0)	-13.19 (6.11)	(-25.90, -0.48)				
		Week 16	10	9 (90.0)	-12.24 (7.13)	(-27.07, 2.59)				
		Week 20	10	8 (80.0)	-6.88 (6.55)	(-20.50, 6.75)				
		Week 26	10	6 (60.0)	-6.82 (6.66)	(-20.94, 7.31)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SICD: Change from baseline in QLQ-C30 Role Functioning (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Female	Peg	Week 4	16	15 (93.8)	14.44 (5.46)	(2.97, 25.92)	30.36 (11.82)	(5.52, 55.20)	0.0194	*
		Week 8	16	14 (87.5)	14.69 (4.55)	(5.09, 24.30)	16.14 (9.73)	(-4.39, 36.68)	0.1154	
		Week 12	16	14 (87.5)	16.80 (4.76)	(6.76, 26.83)	16.08 (10.17)	(-5.38, 37.53)	0.1323	
		Week 16	16	14 (87.5)	11.11 (5.67)	(-0.85, 23.06)	12.59 (12.12)	(-12.97, 38.16)	0.3133	
		Week 20	16	15 (93.8)	11.06 (4.12)	(2.42, 19.71)	9.65 (8.91)	(-9.08, 28.37)	0.2933	
		Week 26	16	13 (81.3)	18.53 (3.17)	(11.78, 25.27)	16.70 (6.86)	(2.08, 31.33)	0.0279	*
	SoC	Week 4	8	7 (87.5)	-15.91 (9.68)	(-36.25, 4.42)				
		Week 8	8	7 (87.5)	-1.45 (7.73)	(-17.77, 14.87)				
		Week 12	8	7 (87.5)	0.72 (8.08)	(-16.33, 17.77)				
		Week 16	8	7 (87.5)	-1.49 (9.63)	(-21.81, 18.83)				
		Week 20	8	7 (87.5)	1.42 (7.30)	(-13.91, 16.74)				
		Week 26	8	6 (75.0)	1.82 (5.59)	(-10.08, 13.73)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SIHD: Change from baseline in QLQ-C30 Role Functioning (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Role Functioning (censored)	Male	Baseline	Peg	19	19 (100.0)	72.81 (29.51)	16.7	83.33	100.0	
			SoC	10	9 (90.0)	77.78 (33.33)	0.0	83.33	100.0	
		Week 4	Peg	19	16 (84.2)	87.50 (14.27)	66.7	91.67	100.0	
			SoC	10	9 (90.0)	75.93 (22.22)	33.3	66.67	100.0	
		Week 8	Peg	19	15 (78.9)	86.67 (19.11)	33.3	100.00	100.0	
			SoC	10	9 (90.0)	77.78 (20.41)	50.0	83.33	100.0	
		Week 12	Peg	19	17 (89.5)	83.33 (17.68)	50.0	83.33	100.0	
			SoC	10	9 (90.0)	74.07 (23.73)	33.3	66.67	100.0	
		Week 16	Peg	19	16 (84.2)	80.21 (21.27)	33.3	83.33	100.0	
			SoC	10	10 (100.0)	70.00 (30.23)	0.0	66.67	100.0	
		Week 20	Peg	19	17 (89.5)	80.39 (22.23)	33.3	83.33	100.0	
			SoC	10	9 (90.0)	77.78 (18.63)	50.0	66.67	100.0	
		Week 26	Peg	19	14 (73.7)	84.52 (20.11)	50.0	100.00	100.0	
			SoC	10	7 (70.0)	78.57 (15.85)	66.7	66.67	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SIHD: Change from baseline in QLQ-C30 Role Functioning (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Role Functioning (censored)	Female	Baseline	Peg	16	16 (100.0)	69.79 (29.95)	0.0	75.00	100.0	
			SoC	8	7 (87.5)	88.10 (12.60)	66.7	83.33	100.0	
		Week 4	Peg	16	15 (93.8)	93.33 (12.28)	66.7	100.00	100.0	
			SoC	8	8 (100.0)	66.67 (30.86)	0.0	66.67	100.0	
		Week 8	Peg	16	14 (87.5)	95.24 (10.19)	66.7	100.00	100.0	
			SoC	8	7 (87.5)	73.81 (23.29)	33.3	66.67	100.0	
		Week 12	Peg	16	14 (87.5)	96.43 (9.65)	66.7	100.00	100.0	
			SoC	8	7 (87.5)	78.57 (24.93)	33.3	83.33	100.0	
		Week 16	Peg	16	14 (87.5)	91.67 (16.98)	50.0	100.00	100.0	
			SoC	8	7 (87.5)	73.81 (23.29)	33.3	66.67	100.0	
		Week 20	Peg	16	15 (93.8)	91.11 (10.67)	66.7	100.00	100.0	
			SoC	8	8 (100.0)	77.08 (23.46)	33.3	75.00	100.0	
		Week 26	Peg	16	13 (81.3)	97.44 (6.26)	83.3	100.00	100.0	
			SoC	8	6 (75.0)	80.56 (16.39)	66.7	75.00	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SIHHD: Change from baseline in QLQ-C30 Role Functioning (censored) by sex
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Male in QLQ-C30 Role Functioning (censored)	Week 4	Peg	19	16 (84.2)	12.50 (32.49)	-33.3	0.00	83.3	0.95 (0.48) [0.10, 2.00]
		SoC	10	8 (80.0)	-14.58 (10.68)	-33.3	-16.67	0.0	
	Week 8	Peg	19	15 (78.9)	7.78 (26.63)	-66.7	0.00	50.0	0.78 (0.48) [-0.08, 1.81]
		SoC	10	8 (80.0)	-12.50 (21.36)	-50.0	-8.33	16.7	
	Week 12	Peg	19	17 (89.5)	6.86 (24.34)	-33.3	0.00	50.0	1.06 (0.48) [0.22, 2.11]
		SoC	10	8 (80.0)	-16.67 (12.60)	-33.3	-16.67	0.0	
	Week 16	Peg	19	16 (84.2)	3.13 (28.03)	-66.7	0.00	50.0	0.56 (0.45) [-0.27, 1.49]
		SoC	10	9 (90.0)	-11.11 (16.67)	-33.3	-16.67	16.7	
	Week 20	Peg	19	17 (89.5)	8.82 (22.91)	-50.0	0.00	50.0	0.97 (0.47) [0.13, 2.00]
		SoC	10	8 (80.0)	-12.50 (17.25)	-33.3	-16.67	16.7	
	Week 26	Peg	19	14 (73.7)	8.33 (25.11)	-50.0	0.00	50.0	0.80 (0.54) [-0.16, 1.97]
		SoC	10	6 (60.0)	-11.11 (17.21)	-33.3	-16.67	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SIHHD: Change from baseline in QLQ-C30 Role Functioning (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Role Functioning (censored)	Female	Week 4	Peg	16	15 (93.8)	18.89 (26.63)	-33.3	16.67	66.7	1.54 (0.55) [0.63, 2.80]
			SoC	8	7 (87.5)	-26.19 (31.71)	-83.3	-33.33	16.7	
		Week 8	Peg	16	14 (87.5)	20.24 (27.09)	-16.7	16.67	66.7	1.24 (0.54) [0.33, 2.45]
			SoC	8	7 (87.5)	-14.29 (26.23)	-50.0	-16.67	16.7	
		Week 12	Peg	16	14 (87.5)	21.43 (25.68)	-16.7	16.67	66.7	1.16 (0.53) [0.26, 2.36]
			SoC	8	7 (87.5)	-9.52 (25.20)	-50.0	0.00	16.7	
		Week 16	Peg	16	14 (87.5)	16.67 (30.66)	-50.0	16.67	66.7	1.01 (0.52) [0.11, 2.17]
			SoC	8	7 (87.5)	-14.29 (26.23)	-50.0	-16.67	16.7	
		Week 20	Peg	16	15 (93.8)	16.67 (25.97)	-16.7	16.67	66.7	1.14 (0.52) [0.25, 2.30]
			SoC	8	7 (87.5)	-14.29 (26.23)	-50.0	-16.67	16.7	
		Week 26	Peg	16	13 (81.3)	23.08 (25.04)	0.0	16.67	66.7	1.23 (0.58) [0.26, 2.53]
			SoC	8	6 (75.0)	-8.33 (22.97)	-33.3	-8.33	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SICB: Change from baseline in QLQ-C30 Role Functioning (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.1893

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SICB: Change from baseline in QLQ-C30 Role Functioning (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Latin America	Peg	Week 4	12	11 (91.7)	29.30 (1.72)	(25.40, 33.20)	22.05 (4.74)	(11.32, 32.78)	0.0012	*
		Week 8	12	10 (83.3)	23.09 (2.69)	(16.90, 29.28)	21.88 (7.05)	(5.62, 38.13)	0.0146	*
		Week 12	12	11 (91.7)	23.98 (4.14)	(14.60, 33.36)	23.29 (11.41)	(-2.51, 49.09)	0.0716	
		Week 16	12	10 (83.3)	18.41 (4.95)	(6.99, 29.83)	18.78 (13.00)	(-11.19, 48.76)	0.1865	
		Week 20	12	10 (83.3)	23.14 (3.88)	(14.20, 32.08)	22.16 (10.30)	(-1.59, 45.92)	0.0636	
		Week 26	12	9 (75.0)	27.58 (3.54)	(19.20, 35.95)	26.15 (8.98)	(4.90, 47.39)	0.0226	*
	SoC	Week 4	2	2 (100.0)	7.25 (4.34)	(-2.57, 17.08)				
		Week 8	2	2 (100.0)	1.21 (6.35)	(-13.43, 15.86)				
		Week 12	2	2 (100.0)	0.69 (10.44)	(-22.92, 24.31)				
		Week 16	2	2 (100.0)	-0.38 (11.71)	(-27.38, 26.63)				
		Week 20	2	2 (100.0)	0.97 (9.26)	(-20.38, 22.33)				
		Week 26	2	2 (100.0)	1.43 (8.09)	(-17.70, 20.56)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SICB: Change from baseline in QLQ-C30 Role Functioning (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Asia Pacific Peg	Peg	Week 4	23	20 (87.0)	3.60 (4.60)	(-5.81, 13.01)	20.60 (7.58)	(5.09, 36.10)	0.0110	*
		Week 8	23	19 (82.6)	3.93 (4.58)	(-5.46, 13.31)	12.23 (7.43)	(-3.00, 27.46)	0.1111	
		Week 12	23	20 (87.0)	4.69 (4.23)	(-3.96, 13.34)	15.76 (6.95)	(1.54, 29.98)	0.0310	*
		Week 16	23	20 (87.0)	2.58 (5.13)	(-7.89, 13.06)	13.41 (8.14)	(-3.21, 30.03)	0.1097	
		Week 20	23	22 (95.7)	4.02 (3.83)	(-3.80, 11.83)	13.33 (6.51)	(0.05, 26.61)	0.0492	*
	Week 26	23	18 (78.3)	6.07 (3.73)	(-1.62, 13.77)	12.29 (6.61)	(-1.35, 25.92)	0.0753		
	SoC	Week 4	16	13 (81.3)	-17.00 (5.81)	(-28.89, -5.11)				
		Week 8	16	13 (81.3)	-8.30 (5.60)	(-19.77, 3.17)				
		Week 12	16	13 (81.3)	-11.07 (5.34)	(-22.00, -0.15)				
		Week 16	16	14 (87.5)	-10.83 (6.16)	(-23.42, 1.76)				
Week 20		16	13 (81.3)	-9.31 (5.08)	(-19.68, 1.05)					
Week 26	16	10 (62.5)	-6.21 (5.33)	(-17.21, 4.79)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SIHB: Change from baseline in QLQ-C30 Role Functioning (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Role Functioning (censored)	Latin America	Baseline	Peg	12	12 (100.0)	59.72 (33.68)	0.0	66.67	100.0	
			SoC	2	2 (100.0)	91.67 (11.79)	83.3	91.67	100.0	
		Week 4	Peg	12	11 (91.7)	98.48 (5.03)	83.3	100.00	100.0	
			SoC	2	2 (100.0)	75.00 (11.79)	66.7	75.00	83.3	
		Week 8	Peg	12	10 (83.3)	96.67 (7.03)	83.3	100.00	100.0	
			SoC	2	2 (100.0)	75.00 (11.79)	66.7	75.00	83.3	
		Week 12	Peg	12	11 (91.7)	92.42 (13.67)	66.7	100.00	100.0	
			SoC	2	2 (100.0)	75.00 (11.79)	66.7	75.00	83.3	
		Week 16	Peg	12	10 (83.3)	91.67 (14.16)	66.7	100.00	100.0	
			SoC	2	2 (100.0)	75.00 (11.79)	66.7	75.00	83.3	
		Week 20	Peg	12	10 (83.3)	91.67 (11.79)	66.7	100.00	100.0	
			SoC	2	2 (100.0)	75.00 (11.79)	66.7	75.00	83.3	
		Week 26	Peg	12	9 (75.0)	96.30 (11.11)	66.7	100.00	100.0	
			SoC	2	2 (100.0)	75.00 (11.79)	66.7	75.00	83.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SIHB: Change from baseline in QLQ-C30 Role Functioning (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Role Functioning (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	77.54 (25.43)	16.7	83.33	100.0	
			SoC	16	14 (87.5)	80.95 (27.62)	0.0	83.33	100.0	
		Week 4	Peg	23	20 (87.0)	85.83 (14.58)	66.7	83.33	100.0	
			SoC	16	15 (93.8)	71.11 (27.79)	0.0	66.67	100.0	
		Week 8	Peg	23	19 (82.6)	87.72 (18.29)	33.3	100.00	100.0	
			SoC	16	14 (87.5)	76.19 (22.37)	33.3	75.00	100.0	
		Week 12	Peg	23	20 (87.0)	87.50 (16.99)	50.0	100.00	100.0	
			SoC	16	14 (87.5)	76.19 (25.08)	33.3	75.00	100.0	
		Week 16	Peg	23	20 (87.0)	82.50 (21.95)	33.3	100.00	100.0	
			SoC	16	15 (93.8)	71.11 (28.50)	0.0	66.67	100.0	
		Week 20	Peg	23	22 (95.7)	82.58 (20.23)	33.3	83.33	100.0	
			SoC	16	15 (93.8)	77.78 (21.52)	33.3	66.67	100.0	
		Week 26	Peg	23	18 (78.3)	87.96 (17.90)	50.0	100.00	100.0	
			SoC	16	11 (68.8)	80.30 (16.36)	66.7	66.67	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SIHB: Change from baseline in QLQ-C30 Role Functioning (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline in QLQ-C30 Role Functioning (censored)	Latin America	Week 4	Peg	12	11 (91.7)	33.33 (29.81)	0.0	33.33	83.3	1.64 (0.95)	[0.13, 3.87]
			SoC	2	2 (100.0)	-16.67 (0.00)	-16.7	-16.67	-16.7		
		Week 8	Peg	12	10 (83.3)	26.67 (26.29)	0.0	16.67	66.7	1.60 (0.97)	[0.09, 3.92]
			SoC	2	2 (100.0)	-16.67 (0.00)	-16.7	-16.67	-16.7		
		Week 12	Peg	12	11 (91.7)	27.27 (27.15)	0.0	16.67	66.7	1.58 (0.94)	[0.07, 3.79]
			SoC	2	2 (100.0)	-16.67 (0.00)	-16.7	-16.67	-16.7		
		Week 16	Peg	12	10 (83.3)	21.67 (28.38)	0.0	8.33	66.7	1.31 (0.94)	[-0.21, 3.50]
			SoC	2	2 (100.0)	-16.67 (0.00)	-16.7	-16.67	-16.7		
		Week 20	Peg	12	10 (83.3)	26.67 (29.61)	-16.7	16.67	66.7	1.42 (0.95)	[-0.10, 3.66]
			SoC	2	2 (100.0)	-16.67 (0.00)	-16.7	-16.67	-16.7		
		Week 26	Peg	12	9 (75.0)	31.48 (28.19)	0.0	33.33	66.7	1.66 (1.02)	[0.13, 4.12]
			SoC	2	2 (100.0)	-16.67 (0.00)	-16.7	-16.67	-16.7		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SIHB: Change from baseline in QLQ-C30 Role Functioning (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Role Functioning (censored)	Asia Pacific	Week 4	Peg	23	20 (87.0)	5.83 (24.94)	-33.3	0.00	66.7	1.03 (0.39) [0.33, 1.89]
			SoC	16	13 (81.3)	-20.51 (24.68)	-83.3	-16.67	16.7	
		Week 8	Peg	23	19 (82.6)	7.02 (25.65)	-66.7	0.00	50.0	0.77 (0.39) [0.06, 1.59]
			SoC	16	13 (81.3)	-12.82 (24.68)	-50.0	0.00	16.7	
		Week 12	Peg	23	20 (87.0)	5.83 (21.81)	-33.3	0.00	50.0	0.85 (0.39) [0.15, 1.67]
			SoC	16	13 (81.3)	-12.82 (20.59)	-50.0	-16.67	16.7	
		Week 16	Peg	23	20 (87.0)	3.33 (28.92)	-66.7	0.00	50.0	0.56 (0.37) [-0.12, 1.33]
			SoC	16	14 (87.5)	-11.90 (22.10)	-50.0	-8.33	16.7	
		Week 20	Peg	23	22 (95.7)	6.06 (18.93)	-50.0	0.00	33.3	0.90 (0.38) [0.22, 1.71]
			SoC	16	13 (81.3)	-12.82 (22.72)	-50.0	-16.67	16.7	
		Week 26	Peg	23	18 (78.3)	7.41 (20.79)	-50.0	0.00	50.0	0.73 (0.42) [-0.04, 1.63]
			SoC	16	10 (62.5)	-8.33 (21.15)	-33.3	-8.33	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SICA: Change from baseline in QLQ-C30 Role Functioning (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.3722

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SICA: Change from baseline in QLQ-C30 Role Functioning (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 4	21	18 (85.7)	18.26 (3.93)	(10.07, 26.46)	24.01 (8.51)	(6.25, 41.76)	0.0106	*
		Week 8	21	17 (81.0)	19.27 (3.07)	(12.86, 25.69)	21.47 (6.47)	(7.93, 35.01)	0.0036	*
		Week 12	21	18 (85.7)	18.87 (3.73)	(11.10, 26.65)	30.15 (8.08)	(13.31, 47.00)	0.0013	*
		Week 16	21	17 (81.0)	18.11 (4.97)	(7.75, 28.47)	27.76 (9.73)	(7.46, 48.05)	0.0098	*
		Week 20	21	18 (85.7)	17.52 (4.07)	(9.04, 26.00)	20.60 (8.90)	(2.04, 39.16)	0.0313	*
	Week 26	21	15 (71.4)	21.60 (3.49)	(14.16, 29.03)	24.02 (8.56)	(5.76, 42.27)	0.0133	*	
	SoC	Week 4	8	5 (62.5)	-5.75 (7.51)	(-21.41, 9.92)				
		Week 8	8	5 (62.5)	-2.19 (5.68)	(-14.08, 9.69)				
		Week 12	8	5 (62.5)	-11.28 (7.12)	(-26.14, 3.58)				
		Week 16	8	6 (75.0)	-9.65 (8.36)	(-27.09, 7.79)				
		Week 20	8	5 (62.5)	-3.08 (7.83)	(-19.41, 13.25)				
Week 26		8	3 (37.5)	-2.42 (7.81)	(-19.07, 14.23)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SICA: Change from baseline in QLQ-C30 Role Functioning (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
>= 4	Peg	Week 4	14	13 (92.9)	5.57 (5.90)	(-6.74, 17.88)	24.47 (9.16)	(5.36, 43.58)	0.0147	*
		Week 8	14	12 (85.7)	0.47 (6.41)	(-12.95, 13.89)	12.70 (9.67)	(-7.54, 32.95)	0.2048	
		Week 12	14	13 (92.9)	1.45 (5.26)	(-9.51, 12.42)	10.01 (8.08)	(-6.84, 26.85)	0.2297	
		Week 16	14	13 (92.9)	-3.92 (6.09)	(-16.62, 8.78)	7.65 (9.35)	(-11.85, 27.16)	0.4227	
		Week 20	14	14 (100.0)	0.72 (4.83)	(-9.33, 10.76)	11.72 (7.62)	(-4.13, 27.57)	0.1390	
		Week 26	14	12 (85.7)	4.08 (4.54)	(-5.46, 13.63)	11.37 (7.02)	(-3.37, 26.11)	0.1223	
	SoC	Week 4	10	10 (100.0)	-18.90 (6.77)	(-33.02, -4.79)				
		Week 8	10	10 (100.0)	-12.23 (7.04)	(-26.97, 2.51)				
		Week 12	10	10 (100.0)	-8.55 (6.01)	(-21.09, 3.99)				
		Week 16	10	10 (100.0)	-11.57 (6.96)	(-26.09, 2.95)				
		Week 20	10	10 (100.0)	-11.00 (5.75)	(-22.95, 0.95)				
		Week 26	10	9 (90.0)	-7.29 (5.26)	(-18.35, 3.76)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SIHA: Change from baseline in QLQ-C30 Role Functioning (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Role Functioning (censored)	< 4	Baseline	Peg	21	21 (100.0)	63.49 (32.33)	0.0	66.67	100.0	
			SoC	8	6 (75.0)	66.67 (36.51)	0.0	83.33	100.0	
		Week 4	Peg	21	18 (85.7)	89.81 (14.16)	66.7	100.00	100.0	
			SoC	8	7 (87.5)	76.19 (25.20)	33.3	66.67	100.0	
		Week 8	Peg	21	17 (81.0)	93.14 (11.87)	66.7	100.00	100.0	
			SoC	8	6 (75.0)	77.78 (20.18)	50.0	75.00	100.0	
		Week 12	Peg	21	18 (85.7)	89.81 (16.31)	50.0	100.00	100.0	
			SoC	8	6 (75.0)	69.44 (26.70)	33.3	66.67	100.0	
		Week 16	Peg	21	17 (81.0)	87.25 (19.12)	50.0	100.00	100.0	
			SoC	8	7 (87.5)	64.29 (33.92)	0.0	66.67	100.0	
		Week 20	Peg	21	18 (85.7)	84.26 (20.98)	33.3	100.00	100.0	
			SoC	8	7 (87.5)	78.57 (20.89)	50.0	66.67	100.0	
		Week 26	Peg	21	15 (71.4)	88.89 (17.44)	50.0	100.00	100.0	
			SoC	8	4 (50.0)	75.00 (16.67)	66.7	66.67	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SIHA: Change from baseline in QLQ-C30 Role Functioning (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Role Functioning (censored)	>= 4	Baseline	Peg	14	14 (100.0)	83.33 (19.61)	50.0	91.67	100.0	
			SoC	10	10 (100.0)	91.67 (11.79)	66.7	100.00	100.0	
		Week 4	Peg	14	13 (92.9)	91.03 (12.94)	66.7	100.00	100.0	
			SoC	10	10 (100.0)	68.33 (27.72)	0.0	66.67	100.0	
		Week 8	Peg	14	12 (85.7)	87.50 (20.26)	33.3	100.00	100.0	
			SoC	10	10 (100.0)	75.00 (22.57)	33.3	75.00	100.0	
		Week 12	Peg	14	13 (92.9)	88.46 (15.79)	66.7	100.00	100.0	
			SoC	10	10 (100.0)	80.00 (21.94)	33.3	83.33	100.0	
		Week 16	Peg	14	13 (92.9)	83.33 (21.52)	33.3	100.00	100.0	
			SoC	10	10 (100.0)	76.67 (21.08)	33.3	75.00	100.0	
		Week 20	Peg	14	14 (100.0)	86.90 (14.88)	50.0	83.33	100.0	
			SoC	10	10 (100.0)	76.67 (21.08)	33.3	75.00	100.0	
		Week 26	Peg	14	12 (85.7)	93.06 (15.01)	50.0	100.00	100.0	
			SoC	10	9 (90.0)	81.48 (15.47)	66.7	83.33	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SIHA: Change from baseline in QLQ-C30 Role Functioning (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in QLQ-C30 Role Functioning (censored)	Week 4	Peg	21	18 (85.7)	20.37 (33.60)	-33.3	16.67	83.3	1.07 (0.56) [0.08, 2.29]
		SoC	8	5 (62.5)	-13.33 (7.45)	-16.7	-16.67	0.0	
	Week 8	Peg	21	17 (81.0)	20.59 (24.67)	-16.7	16.67	66.7	1.11 (0.57) [0.11, 2.36]
		SoC	8	5 (62.5)	-6.67 (19.00)	-33.3	0.00	16.7	
	Week 12	Peg	21	18 (85.7)	20.37 (25.92)	-16.7	16.67	66.7	1.49 (0.59) [0.50, 2.82]
		SoC	8	5 (62.5)	-16.67 (11.79)	-33.3	-16.67	0.0	
	Week 16	Peg	21	17 (81.0)	17.65 (29.15)	-50.0	16.67	66.7	0.93 (0.52) [0.00, 2.07]
		SoC	8	6 (75.0)	-8.33 (17.48)	-33.3	-8.33	16.7	
	Week 20	Peg	21	18 (85.7)	19.44 (24.42)	-16.7	16.67	66.7	1.21 (0.57) [0.22, 2.46]
		SoC	8	5 (62.5)	-10.00 (19.00)	-33.3	-16.67	16.7	
	Week 26	Peg	21	15 (71.4)	22.22 (24.93)	0.0	16.67	66.7	1.09 (0.71) [-0.15, 2.65]
		SoC	8	3 (37.5)	-5.56 (19.25)	-16.7	-16.67	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QRWC_SIHA: Change from baseline in QLQ-C30 Role Functioning (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in QLQ-C30 Role Functioning (censored)	Week 4	Peg	14	13 (92.9)	8.97 (22.17)	-33.3	0.00	50.0	1.27 (0.49) [0.44, 2.37]
		SoC	10	10 (100.0)	-23.33 (27.44)	-83.3	-25.00	16.7	
	Week 8	Peg	14	12 (85.7)	4.17 (28.54)	-66.7	0.00	50.0	0.74 (0.47) [-0.10, 1.76]
		SoC	10	10 (100.0)	-16.67 (24.85)	-50.0	-16.67	16.7	
	Week 12	Peg	14	13 (92.9)	3.85 (22.72)	-33.3	0.00	50.0	0.66 (0.46) [-0.16, 1.64]
		SoC	10	10 (100.0)	-11.67 (22.29)	-50.0	-8.33	16.7	
	Week 16	Peg	14	13 (92.9)	-1.28 (27.61)	-66.7	0.00	50.0	0.52 (0.45) [-0.32, 1.46]
		SoC	10	10 (100.0)	-15.00 (22.84)	-50.0	-16.67	16.7	
	Week 20	Peg	14	14 (100.0)	3.57 (21.86)	-50.0	0.00	33.3	0.81 (0.45) [-0.01, 1.79]
		SoC	10	10 (100.0)	-15.00 (22.84)	-50.0	-16.67	16.7	
	Week 26	Peg	14	12 (85.7)	6.94 (25.08)	-50.0	0.00	50.0	0.75 (0.49) [-0.12, 1.80]
		SoC	10	9 (90.0)	-11.11 (20.41)	-33.3	-16.67	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SICC: Change from baseline in QLQ-C30 Emotional Functioning (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.5144

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SICC: Change from baseline in QLQ-C30 Emotional Functioning (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 60	Peg	Week 4	29	26 (89.7)	8.17 (2.45)	(3.18, 13.16)	5.69 (4.55)	(-3.57, 14.96)	0.2198	
		Week 8	29	24 (82.8)	10.37 (3.32)	(3.60, 17.15)	3.76 (5.99)	(-8.46, 15.99)	0.5347	
		Week 12	29	26 (89.7)	11.41 (3.49)	(4.31, 18.51)	5.82 (6.48)	(-7.36, 18.99)	0.3758	
		Week 16	29	25 (86.2)	12.43 (3.18)	(5.97, 18.90)	6.30 (5.60)	(-5.10, 17.70)	0.2690	
		Week 20	29	27 (93.1)	11.12 (3.09)	(4.84, 17.40)	7.36 (5.82)	(-4.46, 19.18)	0.2144	
	Week 26	29	23 (79.3)	13.76 (3.20)	(7.22, 20.29)	8.92 (5.95)	(-3.24, 21.09)	0.1444		
	SoC	Week 4	13	11 (84.6)	2.48 (3.79)	(-5.23, 10.18)				
		Week 8	13	11 (84.6)	6.61 (4.92)	(-3.42, 16.64)				
		Week 12	13	11 (84.6)	5.59 (5.38)	(-5.36, 16.55)				
		Week 16	13	12 (92.3)	6.13 (4.59)	(-3.20, 15.46)				
		Week 20	13	11 (84.6)	3.76 (4.88)	(-6.15, 13.68)				
	Week 26	13	10 (76.9)	4.83 (4.94)	(-5.27, 14.93)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SICC: Change from baseline in QLQ-C30 Emotional Functioning (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	6.04 (9.78)	(-19.09, 31.17)	14.52 (15.78)	(-26.05, 55.09)	0.3998	
		Week 8	6	5 (83.3)	4.32 (10.34)	(-22.27, 30.91)	6.55 (16.70)	(-36.37, 49.48)	0.7108	
		Week 12	6	5 (83.3)	0.69 (9.37)	(-23.41, 24.79)	2.46 (15.13)	(-36.44, 41.36)	0.8773	
		Week 16	6	5 (83.3)	11.12 (11.35)	(-18.07, 40.31)	23.43 (18.35)	(-23.74, 70.60)	0.2578	
		Week 20	6	5 (83.3)	8.14 (7.45)	(-11.01, 27.28)	12.12 (12.04)	(-18.82, 43.06)	0.3602	
		Week 26	6	4 (66.7)	6.59 (4.09)	(-11.01, 24.19)	-8.33 (8.12)	(-43.27, 26.60)	0.4126	
	SoC	Week 4	5	4 (80.0)	-8.48 (11.50)	(-38.04, 21.08)				
		Week 8	5	4 (80.0)	-2.24 (12.17)	(-33.52, 29.04)				
		Week 12	5	4 (80.0)	-1.76 (11.03)	(-30.11, 26.58)				
		Week 16	5	4 (80.0)	-12.31 (13.35)	(-46.62, 22.00)				
		Week 20	5	4 (80.0)	-3.98 (8.75)	(-26.48, 18.52)				
		Week 26	5	2 (40.0)	14.92 (7.05)	(-15.40, 45.25)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SIHC: Change from baseline in QLQ-C30 Emotional Functioning (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Emotional Functioning (censored)	< 60	Baseline	Peg	29	29 (100.0)	72.99 (22.57)	0.0	83.33	100.0	
			SoC	13	12 (92.3)	76.39 (11.14)	66.7	75.00	100.0	
		Week 4	Peg	29	26 (89.7)	82.69 (16.98)	33.3	83.33	100.0	
			SoC	13	12 (92.3)	79.86 (14.85)	58.3	79.17	100.0	
		Week 8	Peg	29	24 (82.8)	86.11 (17.83)	33.3	91.67	100.0	
			SoC	13	11 (84.6)	81.82 (16.17)	58.3	83.33	100.0	
		Week 12	Peg	29	26 (89.7)	85.90 (18.07)	33.3	91.67	100.0	
			SoC	13	11 (84.6)	81.06 (18.67)	50.0	83.33	100.0	
		Week 16	Peg	29	25 (86.2)	87.67 (15.98)	41.7	91.67	100.0	
			SoC	13	12 (92.3)	81.94 (15.82)	58.3	79.17	100.0	
		Week 20	Peg	29	27 (93.1)	85.19 (17.50)	41.7	91.67	100.0	
			SoC	13	12 (92.3)	81.25 (17.81)	58.3	79.17	100.0	
		Week 26	Peg	29	23 (79.3)	86.23 (16.40)	50.0	91.67	100.0	
			SoC	13	10 (76.9)	79.17 (15.84)	58.3	75.00	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SIHC: Change from baseline in QLQ-C30 Emotional Functioning (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Emotional Functioning (censored)	>= 60	Baseline	Peg	6	6 (100.0)	84.72 (23.22)	41.7	95.83	100.0	
			SoC	5	4 (80.0)	64.58 (38.11)	8.3	79.17	91.7	
	Week 4	Peg	6	5 (83.3)	86.67 (12.64)	75.0	83.33	100.0		
		SoC	5	5 (100.0)	65.00 (41.83)	0.0	75.00	100.0		
	Week 8	Peg	6	5 (83.3)	85.00 (13.69)	66.7	91.67	100.0		
		SoC	5	5 (100.0)	70.00 (41.50)	0.0	83.33	100.0		
	Week 12	Peg	6	5 (83.3)	81.67 (12.36)	66.7	83.33	100.0		
		SoC	5	5 (100.0)	70.00 (41.50)	0.0	83.33	100.0		
	Week 16	Peg	6	5 (83.3)	91.67 (11.79)	75.0	100.00	100.0		
		SoC	5	5 (100.0)	65.00 (43.46)	0.0	83.33	100.0		
	Week 20	Peg	6	5 (83.3)	90.00 (9.13)	75.0	91.67	100.0		
		SoC	5	5 (100.0)	70.00 (41.50)	0.0	83.33	100.0		
	Week 26	Peg	6	4 (66.7)	87.50 (10.76)	75.0	87.50	100.0		
		SoC	5	3 (60.0)	94.44 (9.62)	83.3	100.00	100.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SIHC: Change from baseline in QLQ-C30 Emotional Functioning (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in QLQ-C30 Emotional Functioning (censored)	Week 4	Peg	29	26 (89.7)	8.97 (17.78)	-33.3	8.33	41.7	0.51 (0.38) [-0.20, 1.28]
		SoC	13	11 (84.6)	0.76 (8.70)	-16.7	0.00	8.3	
	Week 8	Peg	29	24 (82.8)	11.46 (23.80)	-58.3	16.67	66.7	0.32 (0.38) [-0.40, 1.09]
		SoC	13	11 (84.6)	4.55 (12.00)	-16.7	0.00	25.0	
	Week 12	Peg	29	26 (89.7)	12.18 (24.75)	-58.3	8.33	66.7	0.37 (0.37) [-0.34, 1.13]
		SoC	13	11 (84.6)	3.79 (14.61)	-16.7	0.00	25.0	
	Week 16	Peg	29	25 (86.2)	12.67 (25.24)	-50.0	8.33	100.0	0.32 (0.36) [-0.38, 1.05]
		SoC	13	12 (92.3)	5.56 (12.48)	-16.7	4.17	25.0	
	Week 20	Peg	29	27 (93.1)	11.73 (22.08)	-50.0	16.67	66.7	0.47 (0.37) [-0.24, 1.23]
		SoC	13	11 (84.6)	2.27 (11.84)	-16.7	0.00	25.0	
	Week 26	Peg	29	23 (79.3)	14.13 (23.36)	-41.7	8.33	83.3	0.47 (0.40) [-0.28, 1.29]
		SoC	13	10 (76.9)	4.17 (11.95)	-16.7	4.17	25.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SIHC: Change from baseline in QLQ-C30 Emotional Functioning (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline >= 60 in QLQ-C30 Emotional Functioning (censored)	Week 4	Peg	6	5 (83.3)	5.00 (24.01)	-25.0	0.00	41.7	0.59	(0.82) [-0.82, 2.45]
		SoC	5	4 (80.0)	-8.33 (13.61)	-25.0	-8.33	8.3		
	Week 8	Peg	6	5 (83.3)	3.33 (26.74)	-16.7	-8.33	50.0	0.23	(0.80) [-1.27, 1.91]
		SoC	5	4 (80.0)	-2.08 (7.98)	-8.3	-4.17	8.3		
	Week 12	Peg	6	5 (83.3)	0.00 (24.30)	-16.7	-8.33	41.7	0.10	(0.79) [-1.45, 1.72]
		SoC	5	4 (80.0)	-2.08 (7.98)	-8.3	-4.17	8.3		
	Week 16	Peg	6	5 (83.3)	8.33 (25.69)	-25.0	0.00	41.7	0.65	(0.83) [-0.74, 2.55]
		SoC	5	4 (80.0)	-8.33 (18.00)	-33.3	-4.17	8.3		
	Week 20	Peg	6	5 (83.3)	6.67 (18.07)	-8.3	0.00	33.3	0.53	(0.82) [-0.89, 2.36]
		SoC	5	4 (80.0)	-2.08 (7.98)	-8.3	-4.17	8.3		
	Week 26	Peg	6	4 (66.7)	8.33 (22.57)	-8.3	0.00	41.7	0.17	(1.23) [-2.10, 2.72]
		SoC	5	2 (40.0)	4.17 (5.89)	0.0	4.17	8.3		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SICD: Change from baseline in QLQ-C30 Emotional Functioning (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.8854

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SICD: Change from baseline in QLQ-C30 Emotional Functioning (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Male	Peg	Week 4	19	16 (84.2)	5.43 (3.30)	(-1.45, 12.31)	10.26 (5.67)	(-1.57, 22.09)	0.0856	
		Week 8	19	15 (78.9)	2.03 (4.67)	(-7.74, 11.80)	5.65 (7.94)	(-10.98, 22.27)	0.4859	
		Week 12	19	17 (89.5)	2.72 (4.67)	(-6.98, 12.42)	7.47 (8.26)	(-9.70, 24.65)	0.3756	
		Week 16	19	16 (84.2)	6.56 (4.54)	(-2.88, 16.00)	10.86 (7.70)	(-5.15, 26.88)	0.1728	
		Week 20	19	17 (89.5)	4.23 (4.19)	(-4.49, 12.95)	6.06 (7.42)	(-9.37, 21.50)	0.4231	
		Week 26	19	14 (73.7)	5.23 (4.17)	(-3.60, 14.06)	6.32 (7.66)	(-9.93, 22.56)	0.4219	
	SoC	Week 4	10	8 (80.0)	-4.83 (4.67)	(-14.56, 4.91)				
		Week 8	10	8 (80.0)	-3.62 (6.42)	(-17.05, 9.82)				
		Week 12	10	8 (80.0)	-4.76 (6.84)	(-18.99, 9.48)				
		Week 16	10	9 (90.0)	-4.30 (6.15)	(-17.09, 8.48)				
		Week 20	10	8 (80.0)	-1.83 (6.15)	(-14.63, 10.97)				
		Week 26	10	6 (60.0)	-1.09 (6.39)	(-14.63, 12.46)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SICD: Change from baseline in QLQ-C30 Emotional Functioning (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Female	Peg	Week 4	16	15 (93.8)	10.65 (4.17)	(1.88, 19.42)	8.42 (8.82)	(-10.12, 26.96)	0.3528	
		Week 8	16	14 (87.5)	17.41 (4.56)	(7.79, 27.02)	4.78 (9.60)	(-15.48, 25.04)	0.6250	
		Week 12	16	14 (87.5)	18.96 (4.82)	(8.80, 29.12)	8.53 (10.15)	(-12.88, 29.94)	0.4124	
		Week 16	16	14 (87.5)	21.11 (5.26)	(10.01, 32.21)	20.58 (11.09)	(-2.81, 43.97)	0.0808	
		Week 20	16	15 (93.8)	18.97 (3.90)	(10.77, 27.17)	15.88 (8.25)	(-1.45, 33.22)	0.0702	
		Week 26	16	13 (81.3)	19.52 (3.01)	(13.10, 25.94)	5.14 (6.56)	(-8.85, 19.13)	0.4455	
	SoC	Week 4	8	7 (87.5)	2.23 (7.31)	(-13.12, 17.59)				
		Week 8	8	7 (87.5)	12.62 (7.70)	(-3.62, 28.87)				
		Week 12	8	7 (87.5)	10.44 (8.14)	(-6.73, 27.60)				
		Week 16	8	7 (87.5)	0.53 (8.89)	(-18.23, 19.28)				
		Week 20	8	7 (87.5)	3.09 (6.83)	(-11.27, 17.44)				
		Week 26	8	6 (75.0)	14.37 (5.35)	(2.98, 25.77)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SIH: Change from baseline in QLQ-C30 Emotional Functioning (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Emotional Functioning (censored)	Male	Baseline	Peg	19	19 (100.0)	80.26 (18.68)	41.7	83.33	100.0	
			SoC	10	9 (90.0)	75.93 (12.11)	66.7	75.00	100.0	
		Week 4	Peg	19	16 (84.2)	85.42 (15.06)	58.3	87.50	100.0	
			SoC	10	9 (90.0)	76.85 (19.89)	50.0	75.00	100.0	
		Week 8	Peg	19	15 (78.9)	83.89 (20.53)	33.3	91.67	100.0	
			SoC	10	9 (90.0)	78.70 (17.24)	58.3	66.67	100.0	
		Week 12	Peg	19	17 (89.5)	82.35 (20.60)	33.3	91.67	100.0	
			SoC	10	9 (90.0)	76.85 (19.44)	50.0	66.67	100.0	
		Week 16	Peg	19	16 (84.2)	88.02 (17.47)	41.7	95.83	100.0	
			SoC	10	10 (100.0)	77.50 (20.43)	41.7	75.00	100.0	
		Week 20	Peg	19	17 (89.5)	83.33 (20.20)	41.7	91.67	100.0	
			SoC	10	9 (90.0)	78.70 (17.24)	58.3	66.67	100.0	
		Week 26	Peg	19	14 (73.7)	82.74 (18.62)	50.0	91.67	100.0	
			SoC	10	7 (70.0)	77.38 (17.16)	58.3	66.67	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SIH: Change from baseline in QLQ-C30 Emotional Functioning (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Emotional Functioning (censored)	Female	Baseline	Peg	16	16 (100.0)	68.75 (26.09)	0.0	79.17	100.0	
			SoC	8	7 (87.5)	70.24 (28.41)	8.3	83.33	91.7	
		Week 4	Peg	16	15 (93.8)	81.11 (17.67)	33.3	83.33	100.0	
			SoC	8	8 (100.0)	73.96 (31.63)	0.0	79.17	100.0	
		Week 8	Peg	16	14 (87.5)	88.10 (12.54)	66.7	91.67	100.0	
			SoC	8	7 (87.5)	77.38 (35.59)	0.0	83.33	100.0	
		Week 12	Peg	16	14 (87.5)	88.69 (11.61)	66.7	91.67	100.0	
			SoC	8	7 (87.5)	78.57 (35.96)	0.0	91.67	100.0	
		Week 16	Peg	16	14 (87.5)	88.69 (12.91)	58.3	91.67	100.0	
			SoC	8	7 (87.5)	76.19 (35.50)	0.0	83.33	100.0	
		Week 20	Peg	16	15 (93.8)	88.89 (10.76)	66.7	91.67	100.0	
			SoC	8	8 (100.0)	77.08 (34.72)	0.0	91.67	100.0	
		Week 26	Peg	16	13 (81.3)	90.38 (10.68)	66.7	91.67	100.0	
			SoC	8	6 (75.0)	88.89 (12.55)	75.0	91.67	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SIH: Change from baseline in QLQ-C30 Emotional Functioning (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Emotional Functioning (censored)	Male	Week 4	Peg	19	16 (84.2)	5.73 (16.31)	-33.3	8.33	33.3	0.56 (0.46) [-0.29, 1.54]
			SoC	10	8 (80.0)	-3.13 (12.55)	-25.0	0.00	8.3	0.13 (0.46) [-0.76, 1.06]
		Week 8	Peg	19	15 (78.9)	1.67 (23.19)	-58.3	0.00	50.0	0.13 (0.46) [-0.76, 1.06]
			SoC	10	8 (80.0)	-1.04 (8.26)	-16.7	0.00	8.3	
		Week 12	Peg	19	17 (89.5)	2.45 (24.07)	-58.3	0.00	50.0	0.26 (0.45) [-0.61, 1.18]
			SoC	10	8 (80.0)	-3.12 (9.90)	-16.7	0.00	8.3	
		Week 16	Peg	19	16 (84.2)	5.21 (21.49)	-50.0	8.33	50.0	0.30 (0.44) [-0.54, 1.20]
			SoC	10	9 (90.0)	-0.93 (16.37)	-33.3	0.00	25.0	
		Week 20	Peg	19	17 (89.5)	3.92 (21.27)	-50.0	0.00	50.0	0.26 (0.45) [-0.60, 1.18]
			SoC	10	8 (80.0)	-1.04 (8.26)	-16.7	0.00	8.3	
		Week 26	Peg	19	14 (73.7)	4.76 (19.26)	-41.7	0.00	41.7	0.27 (0.52) [-0.73, 1.33]
			SoC	10	6 (60.0)	0.00 (9.13)	-16.7	0.00	8.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SIH: Change from baseline in QLQ-C30 Emotional Functioning (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline in QLQ-C30 Emotional Functioning (censored)	Female	Week 4	Peg	16	15 (93.8)	11.11 (20.81)	-25.0	8.33	41.7	0.59 (0.49)	[-0.31, 1.64]
			SoC	8	7 (87.5)	0.00 (8.33)	-8.3	0.00	8.3		
		Week 8	Peg	16	14 (87.5)	19.05 (22.27)	-16.7	16.67	66.7	0.58 (0.50)	[-0.34, 1.64]
			SoC	8	7 (87.5)	7.14 (13.11)	-8.3	8.33	25.0		
		Week 12	Peg	16	14 (87.5)	19.64 (22.79)	-25.0	20.83	66.7	0.53 (0.50)	[-0.39, 1.58]
			SoC	8	7 (87.5)	8.33 (14.43)	-8.3	8.33	25.0		
		Week 16	Peg	16	14 (87.5)	19.64 (27.07)	0.0	8.33	100.0	0.56 (0.50)	[-0.36, 1.62]
			SoC	8	7 (87.5)	5.95 (12.47)	-8.3	8.33	25.0		
		Week 20	Peg	16	15 (93.8)	18.89 (19.02)	-8.3	16.67	66.7	0.84 (0.50)	[-0.06, 1.93]
			SoC	8	7 (87.5)	3.57 (13.49)	-8.3	0.00	25.0		
		Week 26	Peg	16	13 (81.3)	22.44 (23.66)	0.0	16.67	83.3	0.65 (0.54)	[-0.33, 1.80]
			SoC	8	6 (75.0)	8.33 (11.79)	-8.3	8.33	25.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SICB: Change from baseline in QLQ-C30 Emotional Functioning (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.0988

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_SICB: Change from baseline in QLQ-C30 Emotional Functioning (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Latin America	Peg	Week 4	12	11 (91.7)	19.37 (2.88)	(12.84, 25.89)	19.38 (7.41)	(2.63, 36.14)	0.0280	*
		Week 8	12	10 (83.3)	22.96 (2.66)	(16.83, 29.08)	21.07 (6.56)	(5.95, 36.19)	0.0123	*
		Week 12	12	11 (91.7)	24.01 (2.15)	(19.14, 28.88)	20.69 (5.53)	(8.17, 33.20)	0.0046	*
		Week 16	12	10 (83.3)	26.80 (3.55)	(18.62, 34.99)	23.31 (8.76)	(3.11, 43.50)	0.0288	*
		Week 20	12	10 (83.3)	24.32 (3.21)	(16.91, 31.73)	20.92 (7.98)	(2.51, 39.33)	0.0307	*
		Week 26	12	9 (75.0)	26.78 (3.92)	(17.51, 36.05)	19.33 (9.38)	(-2.85, 41.50)	0.0783	
	SoC	Week 4	2	2 (100.0)	-0.02 (6.80)	(-15.40, 15.37)				
		Week 8	2	2 (100.0)	1.88 (5.98)	(-11.90, 15.66)				
		Week 12	2	2 (100.0)	3.32 (5.08)	(-8.17, 14.81)				
		Week 16	2	2 (100.0)	3.50 (7.98)	(-14.92, 21.91)				
		Week 20	2	2 (100.0)	3.40 (7.27)	(-13.36, 20.16)				
		Week 26	2	2 (100.0)	7.46 (8.44)	(-12.49, 27.41)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SICB: Change from baseline in QLQ-C30 Emotional Functioning (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Asia Pacific Peg	Peg	Week 4	23	20 (87.0)	1.52 (3.25)	(-5.12, 8.17)	2.32 (5.44)	(-8.80, 13.44)	0.6727	
		Week 8	23	19 (82.6)	1.78 (3.93)	(-6.27, 9.83)	-3.30 (6.48)	(-16.57, 9.96)	0.6141	
		Week 12	23	20 (87.0)	1.66 (4.26)	(-7.06, 10.38)	-1.70 (7.14)	(-16.30, 12.90)	0.8135	
		Week 16	23	20 (87.0)	4.01 (3.74)	(-3.63, 11.65)	1.41 (6.06)	(-10.97, 13.79)	0.8181	
		Week 20	23	22 (95.7)	4.30 (3.37)	(-2.58, 11.17)	2.55 (5.80)	(-9.28, 14.37)	0.6634	
	Week 26	23	18 (78.3)	5.15 (3.23)	(-1.51, 11.81)	-1.10 (5.97)	(-13.43, 11.23)	0.8552		
	SoC	Week 4	16	13 (81.3)	-0.80 (4.13)	(-9.25, 7.66)				
		Week 8	16	13 (81.3)	5.09 (4.83)	(-4.80, 14.98)				
		Week 12	16	13 (81.3)	3.36 (5.43)	(-7.74, 14.46)				
		Week 16	16	14 (87.5)	2.60 (4.53)	(-6.64, 11.85)				
Week 20		16	13 (81.3)	1.75 (4.50)	(-7.43, 10.93)					
Week 26	16	10 (62.5)	6.25 (4.79)	(-3.64, 16.15)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SIH_B: Change from baseline in QLQ-C30 Emotional Functioning (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Emotional Functioning (censored)	Latin America	Baseline	Peg	12	12 (100.0)	61.81 (30.25)	0.0	54.17	100.0	
			SoC	2	2 (100.0)	75.00 (0.00)	75.0	75.00	75.0	
		Week 4	Peg	12	11 (91.7)	83.33 (20.07)	33.3	83.33	100.0	
			SoC	2	2 (100.0)	70.83 (17.68)	58.3	70.83	83.3	
		Week 8	Peg	12	10 (83.3)	89.17 (13.06)	66.7	91.67	100.0	
			SoC	2	2 (100.0)	70.83 (17.68)	58.3	70.83	83.3	
		Week 12	Peg	12	11 (91.7)	88.64 (10.05)	66.7	91.67	100.0	
			SoC	2	2 (100.0)	70.83 (17.68)	58.3	70.83	83.3	
		Week 16	Peg	12	10 (83.3)	93.33 (9.46)	75.0	100.00	100.0	
			SoC	2	2 (100.0)	70.83 (17.68)	58.3	70.83	83.3	
		Week 20	Peg	12	10 (83.3)	86.67 (13.72)	66.7	87.50	100.0	
			SoC	2	2 (100.0)	70.83 (17.68)	58.3	70.83	83.3	
		Week 26	Peg	12	9 (75.0)	87.04 (11.11)	66.7	91.67	100.0	
			SoC	2	2 (100.0)	70.83 (17.68)	58.3	70.83	83.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SIH_B: Change from baseline in QLQ-C30 Emotional Functioning (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Emotional Functioning (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	81.88 (14.14)	50.0	83.33	100.0	
			SoC	16	14 (87.5)	73.21 (21.73)	8.3	75.00	100.0	
		Week 4	Peg	23	20 (87.0)	83.33 (14.31)	58.3	79.17	100.0	
			SoC	16	15 (93.8)	76.11 (26.51)	0.0	75.00	100.0	
		Week 8	Peg	23	19 (82.6)	84.21 (18.82)	33.3	91.67	100.0	
			SoC	16	14 (87.5)	79.17 (27.10)	0.0	83.33	100.0	
		Week 12	Peg	23	20 (87.0)	83.33 (20.05)	33.3	91.67	100.0	
			SoC	16	14 (87.5)	78.57 (28.25)	0.0	87.50	100.0	
		Week 16	Peg	23	20 (87.0)	85.83 (17.12)	41.7	91.67	100.0	
			SoC	16	15 (93.8)	77.78 (27.94)	0.0	83.33	100.0	
		Week 20	Peg	23	22 (95.7)	85.61 (17.85)	41.7	91.67	100.0	
			SoC	16	15 (93.8)	78.89 (27.25)	0.0	83.33	100.0	
		Week 26	Peg	23	18 (78.3)	86.11 (17.62)	50.0	91.67	100.0	
			SoC	16	11 (68.8)	84.85 (15.28)	66.7	83.33	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SIH_B: Change from baseline in QLQ-C30 Emotional Functioning (censored) by region
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Latin in QLQ-C30 Emotional Functioning (censored)	Week 4	Peg	12	11 (91.7)	20.45 (15.53)	0.0	16.67	41.7	1.46 (0.93) [-0.05, 3.62]
		SoC	2	2 (100.0)	-4.17 (17.68)	-16.7	-4.17	8.3	
	Week 8	Peg	12	10 (83.3)	24.17 (25.89)	-8.3	16.67	66.7	1.04 (0.91) [-0.51, 3.11]
		SoC	2	2 (100.0)	-4.17 (17.68)	-16.7	-4.17	8.3	
	Week 12	Peg	12	11 (91.7)	25.76 (24.28)	-8.3	25.00	66.7	1.17 (0.90) [-0.35, 3.22]
		SoC	2	2 (100.0)	-4.17 (17.68)	-16.7	-4.17	8.3	
	Week 16	Peg	12	10 (83.3)	28.33 (31.23)	0.0	16.67	100.0	1.00 (0.91) [-0.56, 3.05]
		SoC	2	2 (100.0)	-4.17 (17.68)	-16.7	-4.17	8.3	
	Week 20	Peg	12	10 (83.3)	25.83 (22.72)	0.0	25.00	66.7	1.24 (0.93) [-0.29, 3.40]
		SoC	2	2 (100.0)	-4.17 (17.68)	-16.7	-4.17	8.3	
	Week 26	Peg	12	9 (75.0)	29.63 (27.99)	-8.3	25.00	83.3	1.14 (0.95) [-0.41, 3.35]
		SoC	2	2 (100.0)	-4.17 (17.68)	-16.7	-4.17	8.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SIH_B: Change from baseline in QLQ-C30 Emotional Functioning (censored) by region
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]		
Change from baseline Asia in QLQ-C30 Emotional Pacific Functioning (censored)	Week 4	Peg	23	20 (87.0)	1.67 (16.80)	-33.3	4.17	25.0	0.20	(0.37)	[-0.52, 0.94]
		SoC	16	13 (81.3)	-1.28 (10.12)	-25.0	0.00	8.3			
	Week 8	Peg	23	19 (82.6)	2.63 (19.85)	-58.3	0.00	33.3	-0.07	(0.37)	[-0.81, 0.66]
		SoC	16	13 (81.3)	3.85 (10.55)	-8.3	0.00	25.0			
	Week 12	Peg	23	20 (87.0)	1.67 (20.87)	-58.3	8.33	33.3	-0.08	(0.37)	[-0.82, 0.64]
		SoC	16	13 (81.3)	3.21 (12.97)	-16.7	0.00	25.0			
	Week 16	Peg	23	20 (87.0)	3.75 (16.55)	-50.0	8.33	25.0	0.05	(0.36)	[-0.66, 0.76]
		SoC	16	14 (87.5)	2.98 (14.84)	-33.3	0.00	25.0			
	Week 20	Peg	23	22 (95.7)	4.17 (17.20)	-50.0	8.33	25.0	0.15	(0.36)	[-0.56, 0.87]
		SoC	16	13 (81.3)	1.92 (10.29)	-8.3	0.00	25.0			
	Week 26	Peg	23	18 (78.3)	5.09 (14.89)	-41.7	8.33	25.0	-0.05	(0.41)	[-0.87, 0.75]
		SoC	16	10 (62.5)	5.83 (9.66)	-8.3	4.17	25.0			

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SICA: Change from baseline in QLQ-C30 Emotional Functioning (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.4229

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SICA: Change from baseline in QLQ-C30 Emotional Functioning (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 4	21	18 (85.7)	12.31 (2.65)	(6.78, 17.84)	11.62 (5.72)	(-0.30, 23.54)	0.0556	
		Week 8	21	17 (81.0)	16.17 (3.09)	(9.70, 22.64)	14.48 (6.51)	(0.86, 28.10)	0.0384	*
		Week 12	21	18 (85.7)	14.56 (3.36)	(7.56, 21.57)	15.33 (7.24)	(0.23, 30.43)	0.0469	*
		Week 16	21	17 (81.0)	13.52 (3.35)	(6.54, 20.51)	12.95 (6.56)	(-0.73, 26.63)	0.0623	
		Week 20	21	18 (85.7)	12.31 (3.18)	(5.68, 18.95)	9.98 (6.87)	(-4.34, 24.30)	0.1617	
		Week 26	21	15 (71.4)	16.57 (3.22)	(9.71, 23.44)	13.32 (7.89)	(-3.49, 30.14)	0.1119	
	SoC	Week 4	8	5 (62.5)	0.69 (5.05)	(-9.84, 11.22)				
		Week 8	8	5 (62.5)	1.69 (5.72)	(-10.27, 13.65)				
		Week 12	8	5 (62.5)	-0.77 (6.39)	(-14.11, 12.57)				
		Week 16	8	6 (75.0)	0.57 (5.64)	(-11.19, 12.33)				
		Week 20	8	5 (62.5)	2.34 (6.06)	(-10.31, 14.98)				
		Week 26	8	3 (37.5)	3.25 (7.20)	(-12.10, 18.60)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SICA: Change from baseline in QLQ-C30 Emotional Functioning (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	2.79 (4.41)	(-6.42, 12.00)	5.58 (6.74)	(-8.48, 19.64)	0.4176	
		Week 8	14	12 (85.7)	2.09 (5.98)	(-10.42, 14.61)	-0.39 (8.92)	(-19.06, 18.27)	0.9653	
		Week 12	14	13 (92.9)	4.71 (6.10)	(-8.02, 17.44)	1.67 (9.32)	(-17.77, 21.10)	0.8599	
		Week 16	14	13 (92.9)	11.66 (6.23)	(-1.33, 24.65)	10.99 (9.50)	(-8.84, 30.81)	0.2613	
		Week 20	14	14 (100.0)	9.67 (5.07)	(-0.88, 20.23)	9.88 (7.92)	(-6.59, 26.35)	0.2258	
		Week 26	14	12 (85.7)	8.87 (4.52)	(-0.63, 18.36)	4.02 (6.90)	(-10.48, 18.52)	0.5677	
	SoC	Week 4	10	10 (100.0)	-2.79 (5.04)	(-13.31, 7.73)				
		Week 8	10	10 (100.0)	2.49 (6.56)	(-11.23, 16.21)				
		Week 12	10	10 (100.0)	3.04 (6.97)	(-11.49, 17.58)				
		Week 16	10	10 (100.0)	0.68 (7.11)	(-14.15, 15.50)				
		Week 20	10	10 (100.0)	-0.21 (6.02)	(-12.72, 12.30)				
		Week 26	10	9 (90.0)	4.85 (5.22)	(-6.12, 15.81)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SIIHA: Change from baseline in QLQ-C30 Emotional Functioning (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Emotional Functioning (censored)	< 4	Baseline	Peg	21	21 (100.0)	71.43 (20.34)	41.7	66.67	100.0	
			SoC	8	6 (75.0)	75.00 (12.91)	66.7	70.83	100.0	
		Week 4	Peg	21	18 (85.7)	84.26 (12.42)	66.7	83.33	100.0	
			SoC	8	7 (87.5)	82.14 (19.50)	50.0	83.33	100.0	
		Week 8	Peg	21	17 (81.0)	89.71 (14.29)	50.0	100.00	100.0	
			SoC	8	6 (75.0)	80.56 (16.39)	66.7	75.00	100.0	
		Week 12	Peg	21	18 (85.7)	86.57 (14.89)	41.7	91.67	100.0	
			SoC	8	6 (75.0)	77.78 (20.18)	50.0	75.00	100.0	
		Week 16	Peg	21	17 (81.0)	87.25 (14.17)	58.3	91.67	100.0	
			SoC	8	7 (87.5)	78.57 (21.44)	41.7	83.33	100.0	
		Week 20	Peg	21	18 (85.7)	83.80 (16.54)	50.0	91.67	100.0	
			SoC	8	7 (87.5)	83.33 (16.67)	66.7	83.33	100.0	
		Week 26	Peg	21	15 (71.4)	85.00 (16.12)	50.0	91.67	100.0	
			SoC	8	4 (50.0)	79.17 (15.96)	66.7	75.00	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SIIHA: Change from baseline in QLQ-C30 Emotional Functioning (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Emotional Functioning (censored)	>= 4	Baseline	Peg	14	14 (100.0)	80.36 (25.86)	0.0	87.50	100.0	
			SoC	10	10 (100.0)	72.50 (24.23)	8.3	79.17	91.7	
		Week 4	Peg	14	13 (92.9)	82.05 (20.93)	33.3	83.33	100.0	
			SoC	10	10 (100.0)	70.83 (28.67)	0.0	75.00	100.0	
		Week 8	Peg	14	12 (85.7)	80.56 (19.57)	33.3	91.67	100.0	
			SoC	10	10 (100.0)	76.67 (30.88)	0.0	83.33	100.0	
		Week 12	Peg	14	13 (92.9)	83.33 (20.41)	33.3	83.33	100.0	
			SoC	10	10 (100.0)	77.50 (31.19)	0.0	87.50	100.0	
		Week 16	Peg	14	13 (92.9)	89.74 (17.06)	41.7	100.00	100.0	
			SoC	10	10 (100.0)	75.83 (30.79)	0.0	79.17	100.0	
		Week 20	Peg	14	14 (100.0)	88.69 (16.54)	41.7	91.67	100.0	
			SoC	10	10 (100.0)	74.17 (31.29)	0.0	79.17	100.0	
		Week 26	Peg	14	12 (85.7)	88.19 (15.27)	50.0	91.67	100.0	
			SoC	10	9 (90.0)	84.26 (16.37)	58.3	83.33	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SIHA: Change from baseline in QLQ-C30 Emotional Functioning (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in QLQ-C30 Emotional Functioning (censored)	Week 4	Peg	21	18 (85.7)	12.96 (15.71)	-16.7	12.50	41.7	0.92 (0.55) [-0.07, 2.11]
		SoC	8	5 (62.5)	-1.67 (13.69)	-25.0	0.00	8.3	
	Week 8	Peg	21	17 (81.0)	16.67 (18.40)	-16.7	16.67	50.0	0.96 (0.56) [-0.04, 2.18]
		SoC	8	5 (62.5)	0.00 (5.89)	-8.3	0.00	8.3	
	Week 12	Peg	21	18 (85.7)	15.28 (19.65)	-16.7	8.33	50.0	0.99 (0.56) [-0.00, 2.20]
		SoC	8	5 (62.5)	-3.33 (9.50)	-16.7	0.00	8.3	
	Week 16	Peg	21	17 (81.0)	13.73 (15.85)	0.0	8.33	50.0	0.79 (0.52) [-0.14, 1.90]
		SoC	8	6 (75.0)	0.00 (19.00)	-33.3	0.00	25.0	
	Week 20	Peg	21	18 (85.7)	12.96 (18.13)	-16.7	16.67	50.0	0.76 (0.55) [-0.24, 1.92]
		SoC	8	5 (62.5)	0.00 (5.89)	-8.3	0.00	8.3	
	Week 26	Peg	21	15 (71.4)	16.67 (16.67)	0.0	8.33	50.0	0.84 (0.70) [-0.41, 2.35]
		SoC	8	3 (37.5)	2.78 (4.81)	0.0	0.00	8.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QEW_C_SIHA: Change from baseline in QLQ-C30 Emotional Functioning (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline \geq 4 in QLQ-C30 Emotional Functioning (censored)	Week 4	Peg	14	13 (92.9)	1.92 (20.74)	-33.3	0.00	33.3	0.21	(0.44) [-0.65, 1.11]
		SoC	10	10 (100.0)	-1.67 (9.46)	-16.7	-4.17	8.3		
	Week 8	Peg	14	12 (85.7)	0.69 (28.53)	-58.3	0.00	66.7	-0.15	(0.45) [-1.06, 0.73]
		SoC	10	10 (100.0)	4.17 (13.18)	-16.7	4.17	25.0		
	Week 12	Peg	14	13 (92.9)	3.21 (29.76)	-58.3	0.00	66.7	-0.07	(0.44) [-0.95, 0.80]
		SoC	10	10 (100.0)	5.00 (14.27)	-16.7	4.17	25.0		
	Week 16	Peg	14	13 (92.9)	9.62 (33.99)	-50.0	8.33	100.0	0.22	(0.44) [-0.63, 1.13]
		SoC	10	10 (100.0)	3.33 (12.55)	-16.7	4.17	25.0		
	Week 20	Peg	14	14 (100.0)	8.33 (25.32)	-50.0	8.33	66.7	0.30	(0.44) [-0.53, 1.20]
		SoC	10	10 (100.0)	1.67 (12.91)	-16.7	0.00	25.0		
	Week 26	Peg	14	12 (85.7)	9.03 (29.18)	-41.7	4.17	83.3	0.18	(0.47) [-0.72, 1.13]
		SoC	10	9 (90.0)	4.63 (12.58)	-16.7	8.33	25.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SICC: Change from baseline in QLQ-C30 Cognitive Function (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.8896

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SICC: Change from baseline in QLQ-C30 Cognitive Function (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
< 60	Peg	Week 4	29	26 (89.7)	4.61 (2.98)	(-1.46, 10.67)	5.30 (5.61)	(-6.11, 16.71)	0.3516	
		Week 8	29	24 (82.8)	5.43 (3.73)	(-2.19, 13.04)	6.74 (6.84)	(-7.22, 20.69)	0.3325	
		Week 12	29	26 (89.7)	5.93 (3.56)	(-1.31, 13.18)	4.82 (6.67)	(-8.76, 18.40)	0.4751	
		Week 16	29	25 (86.2)	8.58 (3.33)	(1.82, 15.35)	12.56 (5.93)	(0.49, 24.62)	0.0418	*
		Week 20	29	27 (93.1)	3.43 (4.03)	(-4.76, 11.61)	5.78 (7.64)	(-9.74, 21.30)	0.4544	
		Week 26	29	23 (79.3)	7.13 (3.17)	(0.63, 13.62)	11.90 (5.96)	(-0.29, 24.09)	0.0553	
	SoC	Week 4	13	11 (84.6)	-0.69 (4.63)	(-10.12, 8.74)				
		Week 8	13	11 (84.6)	-1.31 (5.57)	(-12.67, 10.06)				
		Week 12	13	11 (84.6)	1.11 (5.52)	(-10.13, 12.35)				
		Week 16	13	12 (92.3)	-3.97 (4.83)	(-13.79, 5.85)				
		Week 20	13	11 (84.6)	-2.35 (6.39)	(-15.33, 10.63)				
		Week 26	13	10 (76.9)	-4.77 (4.93)	(-14.86, 5.31)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SICC: Change from baseline in QLQ-C30 Cognitive Function (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	7.92 (11.28)	(-21.09, 36.93)	29.38 (18.21)	(-17.42, 76.19)	0.1675	
		Week 8	6	5 (83.3)	-0.78 (9.28)	(-24.65, 23.09)	9.83 (14.98)	(-28.68, 48.34)	0.5408	
		Week 12	6	5 (83.3)	-2.79 (8.37)	(-24.30, 18.71)	10.02 (13.50)	(-24.68, 44.72)	0.4913	
		Week 16	6	5 (83.3)	7.85 (6.85)	(-9.75, 25.45)	26.54 (11.16)	(-2.15, 55.22)	0.0633	
		Week 20	6	5 (83.3)	9.61 (8.07)	(-11.14, 30.36)	25.88 (13.16)	(-7.94, 59.70)	0.1063	
		Week 26	6	4 (66.7)	-0.46 (10.01)	(-43.53, 42.61)	-2.78 (20.60)	(-91.41, 85.86)	0.9051	
	SoC	Week 4	5	4 (80.0)	-21.46 (13.31)	(-55.69, 12.76)				
		Week 8	5	4 (80.0)	-10.61 (10.96)	(-38.77, 17.55)				
		Week 12	5	4 (80.0)	-12.81 (9.87)	(-38.19, 12.56)				
		Week 16	5	4 (80.0)	-18.68 (8.07)	(-39.43, 2.06)				
		Week 20	5	4 (80.0)	-16.26 (9.51)	(-40.72, 8.19)				
		Week 26	5	2 (40.0)	2.31 (16.93)	(-70.54, 75.16)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SIHC: Change from baseline in QLQ-C30 Cognitive Function (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Cognitive Function (censored)	< 60	Baseline	Peg	29	29 (100.0)	77.59 (21.02)	33.3	83.33	100.0	
			SoC	13	12 (92.3)	86.11 (15.62)	50.0	83.33	100.0	
		Week 4	Peg	29	26 (89.7)	84.62 (19.39)	16.7	83.33	100.0	
			SoC	13	12 (92.3)	83.33 (15.89)	50.0	83.33	100.0	
		Week 8	Peg	29	24 (82.8)	84.72 (20.21)	16.7	91.67	100.0	
			SoC	13	11 (84.6)	81.82 (18.94)	50.0	83.33	100.0	
		Week 12	Peg	29	26 (89.7)	85.90 (20.38)	16.7	100.00	100.0	
			SoC	13	11 (84.6)	84.85 (18.94)	50.0	100.00	100.0	
		Week 16	Peg	29	25 (86.2)	88.67 (17.82)	33.3	100.00	100.0	
			SoC	13	12 (92.3)	79.17 (17.59)	50.0	75.00	100.0	
		Week 20	Peg	29	27 (93.1)	84.57 (23.08)	16.7	100.00	100.0	
			SoC	13	12 (92.3)	83.33 (17.41)	50.0	83.33	100.0	
		Week 26	Peg	29	23 (79.3)	86.96 (17.38)	50.0	100.00	100.0	
			SoC	13	10 (76.9)	78.33 (17.66)	50.0	75.00	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SIHC: Change from baseline in QLQ-C30 Cognitive Function (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Cognitive Function (censored)	>= 60	Baseline	Peg	6	6 (100.0)	83.33 (18.26)	50.0	83.33	100.0	
			SoC	5	4 (80.0)	91.67 (16.67)	66.7	100.00	100.0	
		Week 4	Peg	6	5 (83.3)	93.33 (9.13)	83.3	100.00	100.0	
			SoC	5	5 (100.0)	73.33 (34.56)	16.7	83.33	100.0	
		Week 8	Peg	6	5 (83.3)	86.67 (13.94)	66.7	83.33	100.0	
			SoC	5	5 (100.0)	80.00 (21.73)	50.0	83.33	100.0	
		Week 12	Peg	6	5 (83.3)	83.33 (11.79)	66.7	83.33	100.0	
			SoC	5	5 (100.0)	80.00 (21.73)	50.0	83.33	100.0	
		Week 16	Peg	6	5 (83.3)	90.00 (9.13)	83.3	83.33	100.0	
			SoC	5	5 (100.0)	76.67 (22.36)	50.0	66.67	100.0	
		Week 20	Peg	6	5 (83.3)	93.33 (9.13)	83.3	100.00	100.0	
			SoC	5	5 (100.0)	76.67 (22.36)	50.0	66.67	100.0	
		Week 26	Peg	6	4 (66.7)	83.33 (13.61)	66.7	83.33	100.0	
			SoC	5	3 (60.0)	88.89 (19.25)	66.7	100.00	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SIHC: Change from baseline in QLQ-C30 Cognitive Function (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in QLQ-C30 Cognitive Function (censored)	Week 4	Peg	29	26 (89.7)	6.41 (20.04)	-50.0	0.00	50.0	0.61 (0.38) [-0.09, 1.40]
		SoC	13	11 (84.6)	-4.55 (7.78)	-16.7	0.00	0.0	
	Week 8	Peg	29	24 (82.8)	6.94 (23.01)	-50.0	0.00	50.0	0.56 (0.38) [-0.16, 1.35]
		SoC	13	11 (84.6)	-4.55 (10.78)	-16.7	0.00	16.7	
	Week 12	Peg	29	26 (89.7)	7.05 (22.20)	-50.0	0.00	50.0	0.42 (0.37) [-0.29, 1.19]
		SoC	13	11 (84.6)	-1.52 (11.68)	-16.7	0.00	16.7	
	Week 16	Peg	29	25 (86.2)	10.00 (22.57)	-33.3	0.00	66.7	0.84 (0.38) [0.15, 1.64]
		SoC	13	12 (92.3)	-6.94 (11.14)	-16.7	-8.33	16.7	
	Week 20	Peg	29	27 (93.1)	4.32 (25.98)	-66.7	0.00	50.0	0.38 (0.37) [-0.33, 1.14]
		SoC	13	11 (84.6)	-4.55 (10.78)	-16.7	0.00	16.7	
	Week 26	Peg	29	23 (79.3)	7.97 (19.38)	-16.7	0.00	50.0	0.82 (0.41) [0.07, 1.68]
		SoC	13	10 (76.9)	-6.67 (11.65)	-16.7	-8.33	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QTWC_SIHC: Change from baseline in QLQ-C30 Cognitive Function (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in QLQ-C30 Cognitive Function (censored)	Week 4	Peg	6	5 (83.3)	10.00 (22.36)	0.0	0.00	50.0	1.41 (0.95) [0.10, 3.80]
		SoC	5	4 (80.0)	-25.00 (21.52)	-50.0	-25.00	0.0	
	Week 8	Peg	6	5 (83.3)	3.33 (29.81)	-33.3	0.00	50.0	0.73 (0.84) [-0.64, 2.68]
		SoC	5	4 (80.0)	-16.67 (13.61)	-33.3	-16.67	0.0	
	Week 12	Peg	6	5 (83.3)	0.00 (23.57)	-33.3	0.00	33.3	0.74 (0.84) [-0.63, 2.69]
		SoC	5	4 (80.0)	-16.67 (13.61)	-33.3	-16.67	0.0	
	Week 16	Peg	6	5 (83.3)	10.00 (14.91)	0.0	0.00	33.3	1.78 (1.03) [0.46, 4.44]
		SoC	5	4 (80.0)	-20.83 (15.96)	-33.3	-25.00	0.0	
	Week 20	Peg	6	5 (83.3)	13.33 (21.73)	0.0	0.00	50.0	1.56 (0.98) [0.25, 4.05]
		SoC	5	4 (80.0)	-20.83 (15.96)	-33.3	-25.00	0.0	
	Week 26	Peg	6	4 (66.7)	4.17 (20.97)	-16.7	0.00	33.3	0.77 (1.33) [-1.15, 3.94]
		SoC	5	2 (40.0)	-16.67 (23.57)	-33.3	-16.67	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SICD: Change from baseline in QLQ-C30 Cognitive Function (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.4880

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QTWC_SICD: Change from baseline in QLQ-C30 Cognitive Function (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Male	Peg	Week 4	19	16 (84.2)	2.26 (3.75)	(-5.55, 10.08)	12.11 (6.47)	(-1.39, 25.61)	0.0761	
		Week 8	19	15 (78.9)	-4.60 (4.01)	(-13.00, 3.79)	4.36 (6.82)	(-9.92, 18.64)	0.5306	
		Week 12	19	17 (89.5)	-3.95 (4.00)	(-12.27, 4.37)	0.37 (7.09)	(-14.39, 15.12)	0.9593	
		Week 16	19	16 (84.2)	2.07 (3.28)	(-4.75, 8.89)	14.01 (5.55)	(2.47, 25.55)	0.0197	*
		Week 20	19	17 (89.5)	-5.84 (4.94)	(-16.10, 4.42)	2.07 (8.75)	(-16.12, 20.26)	0.8155	
		Week 26	19	14 (73.7)	-2.76 (2.72)	(-8.53, 3.02)	4.70 (5.02)	(-5.94, 15.34)	0.3632	
	SoC	Week 4	10	8 (80.0)	-9.85 (5.33)	(-20.97, 1.28)				
		Week 8	10	8 (80.0)	-8.96 (5.55)	(-20.58, 2.65)				
		Week 12	10	8 (80.0)	-4.32 (5.89)	(-16.57, 7.92)				
		Week 16	10	9 (90.0)	-11.94 (4.47)	(-21.24, -2.63)				
		Week 20	10	8 (80.0)	-7.91 (7.26)	(-23.00, 7.19)				
		Week 26	10	6 (60.0)	-7.46 (4.18)	(-16.32, 1.41)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QTWC_SICD: Change from baseline in QLQ-C30 Cognitive Function (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Female	Peg	Week 4	16	15 (93.8)	6.47 (5.12)	(-4.28, 17.22)	5.32 (11.91)	(-19.69, 30.33)	0.6603	
		Week 8	16	14 (87.5)	11.04 (4.81)	(0.89, 21.19)	2.31 (11.30)	(-21.53, 26.16)	0.8401	
		Week 12	16	14 (87.5)	11.83 (4.39)	(2.56, 21.10)	5.09 (10.32)	(-16.69, 26.87)	0.6281	
		Week 16	16	14 (87.5)	13.90 (4.85)	(3.67, 24.14)	12.96 (11.40)	(-11.09, 37.02)	0.2713	
		Week 20	16	15 (93.8)	13.39 (3.91)	(5.18, 21.61)	8.65 (9.10)	(-10.46, 27.77)	0.3541	
		Week 26	16	13 (81.3)	11.31 (4.25)	(2.25, 20.36)	3.30 (10.40)	(-18.88, 25.48)	0.7554	
	SoC	Week 4	8	7 (87.5)	1.15 (9.60)	(-19.01, 21.32)				
		Week 8	8	7 (87.5)	8.72 (8.77)	(-9.77, 27.22)				
		Week 12	8	7 (87.5)	6.74 (8.01)	(-10.16, 23.63)				
		Week 16	8	7 (87.5)	0.94 (8.84)	(-17.72, 19.60)				
		Week 20	8	7 (87.5)	4.74 (7.33)	(-10.67, 20.15)				
		Week 26	8	6 (75.0)	8.01 (8.21)	(-9.50, 25.52)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SIHD: Change from baseline in QLQ-C30 Cognitive Function (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Cognitive Function (censored)	Male	Baseline	Peg	19	19 (100.0)	86.84 (14.25)	50.0	83.33	100.0	
			SoC	10	9 (90.0)	87.04 (16.20)	50.0	83.33	100.0	
		Week 4	Peg	19	16 (84.2)	89.58 (20.97)	16.7	100.00	100.0	
			SoC	10	9 (90.0)	81.48 (17.57)	50.0	83.33	100.0	
		Week 8	Peg	19	15 (78.9)	82.22 (23.12)	16.7	83.33	100.0	
			SoC	10	9 (90.0)	81.48 (19.44)	50.0	83.33	100.0	
		Week 12	Peg	19	17 (89.5)	83.33 (23.57)	16.7	100.00	100.0	
			SoC	10	9 (90.0)	85.19 (19.44)	50.0	100.00	100.0	
		Week 16	Peg	19	16 (84.2)	88.54 (18.97)	33.3	100.00	100.0	
			SoC	10	10 (100.0)	78.33 (19.33)	50.0	66.67	100.0	
		Week 20	Peg	19	17 (89.5)	82.35 (27.30)	16.7	100.00	100.0	
			SoC	10	9 (90.0)	81.48 (19.44)	50.0	83.33	100.0	
		Week 26	Peg	19	14 (73.7)	85.71 (19.46)	50.0	100.00	100.0	
			SoC	10	7 (70.0)	78.57 (20.89)	50.0	66.67	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SIHD: Change from baseline in QLQ-C30 Cognitive Function (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Cognitive Function (censored)	Female	Baseline	Peg	16	16 (100.0)	68.75 (22.67)	33.3	83.33	100.0	
			SoC	8	7 (87.5)	88.10 (15.85)	66.7	100.00	100.0	
		Week 4	Peg	16	15 (93.8)	82.22 (14.73)	66.7	83.33	100.0	
			SoC	8	8 (100.0)	79.17 (27.82)	16.7	83.33	100.0	
		Week 8	Peg	16	14 (87.5)	88.10 (13.76)	66.7	91.67	100.0	
			SoC	8	7 (87.5)	80.95 (20.25)	50.0	83.33	100.0	
		Week 12	Peg	16	14 (87.5)	88.10 (12.10)	66.7	83.33	100.0	
			SoC	8	7 (87.5)	80.95 (20.25)	50.0	83.33	100.0	
		Week 16	Peg	16	14 (87.5)	89.29 (14.03)	66.7	100.00	100.0	
			SoC	8	7 (87.5)	78.57 (18.54)	50.0	83.33	100.0	
		Week 20	Peg	16	15 (93.8)	90.00 (12.28)	66.7	100.00	100.0	
			SoC	8	8 (100.0)	81.25 (18.77)	50.0	83.33	100.0	
		Week 26	Peg	16	13 (81.3)	87.18 (13.87)	66.7	83.33	100.0	
			SoC	8	6 (75.0)	83.33 (14.91)	66.7	83.33	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SIHHD: Change from baseline in QLQ-C30 Cognitive Function (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Cognitive Function (censored)	Male	Week 4	Peg	19	16 (84.2)	3.12 (17.45)	-50.0	0.00	33.3	0.69 (0.47) [-0.16, 1.69]
			SoC	10	8 (80.0)	-8.33 (12.60)	-33.3	0.00	0.0	
		Week 8	Peg	19	15 (78.9)	-4.44 (17.21)	-50.0	0.00	16.7	0.25 (0.46) [-0.64, 1.19]
			SoC	10	8 (80.0)	-8.33 (8.91)	-16.7	-8.33	0.0	
		Week 12	Peg	19	17 (89.5)	-3.92 (17.21)	-50.0	0.00	16.7	0.02 (0.45) [-0.87, 0.90]
			SoC	10	8 (80.0)	-4.17 (11.79)	-16.7	0.00	16.7	
		Week 16	Peg	19	16 (84.2)	2.08 (13.44)	-33.3	0.00	16.7	0.99 (0.46) [0.17, 2.00]
			SoC	10	9 (90.0)	-11.11 (11.79)	-33.3	-16.67	0.0	
		Week 20	Peg	19	17 (89.5)	-5.88 (22.00)	-66.7	0.00	16.7	0.12 (0.45) [-0.75, 1.02]
			SoC	10	8 (80.0)	-8.33 (12.60)	-33.3	0.00	0.0	
		Week 26	Peg	19	14 (73.7)	-2.38 (11.05)	-16.7	0.00	16.7	0.54 (0.53) [-0.43, 1.65]
			SoC	10	6 (60.0)	-8.33 (9.13)	-16.7	-8.33	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QTWC_SIHD: Change from baseline in QLQ-C30 Cognitive Function (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Cognitive Function (censored)	Female	Week 4	Peg	16	15 (93.8)	11.11 (22.42)	-16.7	0.00	50.0	1.04 (0.52) [0.14, 2.17]
			SoC	8	7 (87.5)	-11.90 (18.54)	-50.0	0.00	0.0	
		Week 8	Peg	16	14 (87.5)	17.86 (24.86)	-16.7	16.67	50.0	1.07 (0.53) [0.16, 2.23]
			SoC	8	7 (87.5)	-7.14 (16.27)	-33.3	0.00	16.7	
		Week 12	Peg	16	14 (87.5)	17.86 (22.13)	-16.7	16.67	50.0	1.17 (0.53) [0.27, 2.37]
			SoC	8	7 (87.5)	-7.14 (16.27)	-33.3	0.00	16.7	
		Week 16	Peg	16	14 (87.5)	19.05 (25.20)	-16.7	16.67	66.7	1.21 (0.54) [0.30, 2.41]
			SoC	8	7 (87.5)	-9.52 (16.27)	-33.3	-16.67	16.7	
		Week 20	Peg	16	15 (93.8)	18.89 (22.60)	-16.7	16.67	50.0	1.31 (0.53) [0.41, 2.51]
			SoC	8	7 (87.5)	-9.52 (16.27)	-33.3	-16.67	16.7	
		Week 26	Peg	16	13 (81.3)	17.95 (20.93)	-16.7	16.67	50.0	1.26 (0.58) [0.29, 2.57]
			SoC	8	6 (75.0)	-8.33 (17.48)	-33.3	-8.33	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QTWC_SICB: Change from baseline in QLQ-C30 Cognitive Function (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.6849

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SICB: Change from baseline in QLQ-C30 Cognitive Function (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Latin America	Peg	Week 4	12	11 (91.7)	18.67 (2.29)	(13.50, 23.85)	16.63 (6.03)	(2.99, 30.26)	0.0222	*
		Week 8	12	10 (83.3)	19.38 (3.76)	(10.71, 28.04)	16.26 (9.58)	(-5.82, 38.34)	0.1280	
		Week 12	12	11 (91.7)	16.03 (3.45)	(8.22, 23.84)	13.66 (9.09)	(-6.91, 34.24)	0.1673	
		Week 16	12	10 (83.3)	19.23 (4.40)	(9.09, 29.36)	15.36 (11.20)	(-10.47, 41.19)	0.2076	
		Week 20	12	10 (83.3)	16.05 (4.61)	(5.41, 26.68)	12.95 (11.64)	(-13.90, 39.80)	0.2984	
		Week 26	12	9 (75.0)	13.75 (4.82)	(2.35, 25.15)	11.07 (11.76)	(-16.73, 38.87)	0.3777	
	SoC	Week 4	2	2 (100.0)	2.05 (5.51)	(-10.41, 14.50)				
		Week 8	2	2 (100.0)	3.12 (8.68)	(-16.89, 23.12)				
		Week 12	2	2 (100.0)	2.37 (8.31)	(-16.43, 21.17)				
		Week 16	2	2 (100.0)	3.87 (10.15)	(-19.53, 27.27)				
		Week 20	2	2 (100.0)	3.10 (10.57)	(-21.27, 27.46)				
		Week 26	2	2 (100.0)	2.68 (10.50)	(-22.16, 27.51)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SICB: Change from baseline in QLQ-C30 Cognitive Function (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Asia Pacific Peg	Peg	Week 4	23	20 (87.0)	-1.55 (3.65)	(-9.01, 5.90)	7.33 (6.03)	(-4.99, 19.65)	0.2335	
		Week 8	23	19 (82.6)	-2.35 (4.27)	(-11.10, 6.39)	4.46 (6.96)	(-9.79, 18.71)	0.5265	
		Week 12	23	20 (87.0)	-1.13 (4.23)	(-9.77, 7.51)	3.54 (6.96)	(-10.69, 17.78)	0.6145	
		Week 16	23	20 (87.0)	3.80 (3.66)	(-3.66, 11.27)	14.00 (5.82)	(2.11, 25.89)	0.0225	*
		Week 20	23	22 (95.7)	-0.13 (4.50)	(-9.32, 9.06)	8.77 (7.64)	(-6.82, 24.35)	0.2601	
	Week 26	23	18 (78.3)	2.38 (3.65)	(-5.16, 9.91)	10.08 (6.48)	(-3.30, 23.46)	0.1331		
	SoC	Week 4	16	13 (81.3)	-8.89 (4.62)	(-18.33, 0.55)				
		Week 8	16	13 (81.3)	-6.82 (5.22)	(-17.52, 3.89)				
		Week 12	16	13 (81.3)	-4.67 (5.34)	(-15.60, 6.25)				
		Week 16	16	14 (87.5)	-10.20 (4.40)	(-19.18, -1.22)				
Week 20		16	13 (81.3)	-8.90 (5.98)	(-21.09, 3.30)					
Week 26	16	10 (62.5)	-7.70 (5.22)	(-18.49, 3.08)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SIH: Change from baseline in QLQ-C30 Cognitive Function (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Cognitive Function (censored)	Latin America	Baseline	Peg	12	12 (100.0)	68.06 (28.83)	33.3	66.67	100.0	
			SoC	2	2 (100.0)	91.67 (11.79)	83.3	91.67	100.0	
		Week 4	Peg	12	11 (91.7)	92.42 (13.67)	66.7	100.00	100.0	
			SoC	2	2 (100.0)	83.33 (23.57)	66.7	83.33	100.0	
		Week 8	Peg	12	10 (83.3)	91.67 (14.16)	66.7	100.00	100.0	
			SoC	2	2 (100.0)	83.33 (23.57)	66.7	83.33	100.0	
		Week 12	Peg	12	11 (91.7)	89.39 (13.48)	66.7	100.00	100.0	
			SoC	2	2 (100.0)	83.33 (23.57)	66.7	83.33	100.0	
		Week 16	Peg	12	10 (83.3)	91.67 (14.16)	66.7	100.00	100.0	
			SoC	2	2 (100.0)	83.33 (23.57)	66.7	83.33	100.0	
		Week 20	Peg	12	10 (83.3)	91.67 (14.16)	66.7	100.00	100.0	
			SoC	2	2 (100.0)	83.33 (23.57)	66.7	83.33	100.0	
		Week 26	Peg	12	9 (75.0)	87.04 (16.20)	66.7	100.00	100.0	
			SoC	2	2 (100.0)	83.33 (23.57)	66.7	83.33	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SIH: Change from baseline in QLQ-C30 Cognitive Function (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Cognitive Function (censored)	Asia	Baseline	Peg	23	23 (100.0)	84.06 (11.76)	50.0	83.33	100.0	
			SoC	16	14 (87.5)	86.90 (16.25)	50.0	91.67	100.0	
	Pacific	Week 4	Peg	23	20 (87.0)	82.50 (19.85)	16.7	83.33	100.0	
			SoC	16	15 (93.8)	80.00 (22.89)	16.7	83.33	100.0	
		Week 8	Peg	23	19 (82.6)	81.58 (20.71)	16.7	83.33	100.0	
			SoC	16	14 (87.5)	80.95 (19.46)	50.0	83.33	100.0	
		Week 12	Peg	23	20 (87.0)	83.33 (21.63)	16.7	83.33	100.0	
			SoC	16	14 (87.5)	83.33 (19.61)	50.0	91.67	100.0	
		Week 16	Peg	23	20 (87.0)	87.50 (17.83)	33.3	100.00	100.0	
			SoC	16	15 (93.8)	77.78 (18.54)	50.0	66.67	100.0	
		Week 20	Peg	23	22 (95.7)	83.33 (24.12)	16.7	83.33	100.0	
			SoC	16	15 (93.8)	81.11 (18.76)	50.0	83.33	100.0	
		Week 26	Peg	23	18 (78.3)	86.11 (17.39)	50.0	91.67	100.0	
			SoC	16	11 (68.8)	80.30 (17.98)	50.0	83.33	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QTWC_SIHB: Change from baseline in QLQ-C30 Cognitive Function (censored) by region
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Latin in QLQ-C30 Cognitive Function (censored)	Week 4	Peg	12	11 (91.7)	21.21 (19.85)	0.0	16.67	50.0	1.43 (0.93) [-0.08, 3.58]
		SoC	2	2 (100.0)	-8.33 (11.79)	-16.7	-8.33	0.0	
	Week 8	Peg	12	10 (83.3)	21.67 (22.29)	0.0	16.67	50.0	1.29 (0.94) [-0.24, 3.46]
		SoC	2	2 (100.0)	-8.33 (11.79)	-16.7	-8.33	0.0	
	Week 12	Peg	12	11 (91.7)	18.18 (20.35)	0.0	16.67	50.0	1.25 (0.91) [-0.27, 3.33]
		SoC	2	2 (100.0)	-8.33 (11.79)	-16.7	-8.33	0.0	
	Week 16	Peg	12	10 (83.3)	21.67 (23.64)	0.0	16.67	66.7	1.22 (0.93) [-0.31, 3.36]
		SoC	2	2 (100.0)	-8.33 (11.79)	-16.7	-8.33	0.0	
	Week 20	Peg	12	10 (83.3)	18.33 (25.40)	-16.7	8.33	50.0	1.01 (0.91) [-0.54, 3.07]
		SoC	2	2 (100.0)	-8.33 (11.79)	-16.7	-8.33	0.0	
	Week 26	Peg	12	9 (75.0)	16.67 (22.05)	-16.7	16.67	50.0	1.08 (0.94) [-0.48, 3.26]
		SoC	2	2 (100.0)	-8.33 (11.79)	-16.7	-8.33	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QTWC_SIHB: Change from baseline in QLQ-C30 Cognitive Function (censored) by region
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Asia in QLQ-C30 Cognitive Pacific Function (censored)	Week 4	Peg	23	20 (87.0)	-0.83 (15.74)	-50.0	0.00	16.7	0.58 (0.38) [-0.12, 1.36]
		SoC	16	13 (81.3)	-10.26 (16.01)	-50.0	0.00	0.0	
	Week 8	Peg	23	19 (82.6)	-1.75 (20.71)	-50.0	0.00	50.0	0.32 (0.38) [-0.39, 1.09]
		SoC	16	13 (81.3)	-7.69 (12.94)	-33.3	0.00	16.7	
	Week 12	Peg	23	20 (87.0)	-0.83 (20.57)	-50.0	0.00	50.0	0.23 (0.37) [-0.48, 0.97]
		SoC	16	13 (81.3)	-5.13 (14.25)	-33.3	0.00	16.7	
	Week 16	Peg	23	20 (87.0)	4.17 (17.83)	-33.3	0.00	50.0	0.89 (0.38) [0.20, 1.69]
		SoC	16	14 (87.5)	-10.71 (14.03)	-33.3	-16.67	16.7	
	Week 20	Peg	23	22 (95.7)	0.00 (23.57)	-66.7	0.00	50.0	0.42 (0.37) [-0.27, 1.17]
		SoC	16	13 (81.3)	-8.97 (14.62)	-33.3	0.00	16.7	
	Week 26	Peg	23	18 (78.3)	2.78 (16.42)	-16.7	0.00	50.0	0.69 (0.42) [-0.09, 1.58]
		SoC	16	10 (62.5)	-8.33 (14.16)	-33.3	-8.33	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SICA: Change from baseline in QLQ-C30 Cognitive Function (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.5653

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SICA: Change from baseline in QLQ-C30 Cognitive Function (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 4	21	18 (85.7)	10.16 (3.22)	(3.45, 16.88)	13.42 (6.97)	(-1.11, 27.96)	0.0683	
		Week 8	21	17 (81.0)	5.92 (3.85)	(-2.14, 13.99)	9.39 (8.16)	(-7.68, 26.46)	0.2640	
		Week 12	21	18 (85.7)	5.77 (3.61)	(-1.77, 13.31)	9.89 (7.82)	(-6.42, 26.20)	0.2206	
		Week 16	21	17 (81.0)	8.85 (3.26)	(2.05, 15.65)	17.25 (6.46)	(3.78, 30.72)	0.0147	*
		Week 20	21	18 (85.7)	3.79 (5.02)	(-6.68, 14.26)	10.77 (10.85)	(-11.86, 33.39)	0.3329	
	Week 26	21	15 (71.4)	8.06 (3.87)	(-0.18, 16.30)	15.04 (9.50)	(-5.20, 35.28)	0.1340		
	SoC	Week 4	8	5 (62.5)	-3.26 (6.15)	(-16.08, 9.57)				
		Week 8	8	5 (62.5)	-3.47 (7.15)	(-18.43, 11.50)				
		Week 12	8	5 (62.5)	-4.11 (6.90)	(-18.51, 10.28)				
		Week 16	8	6 (75.0)	-8.40 (5.53)	(-19.93, 3.13)				
		Week 20	8	5 (62.5)	-6.98 (9.58)	(-26.95, 13.00)				
Week 26	8	3 (37.5)	-6.98 (8.66)	(-25.44, 11.49)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SICA: Change from baseline in QLQ-C30 Cognitive Function (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	-0.07 (5.23)	(-10.98, 10.84)	9.83 (8.11)	(-7.09, 26.76)	0.2398	
		Week 8	14	12 (85.7)	2.61 (5.96)	(-9.87, 15.09)	7.41 (9.05)	(-11.54, 26.35)	0.4233	
		Week 12	14	13 (92.9)	2.73 (5.66)	(-9.07, 14.54)	4.62 (8.73)	(-13.59, 22.83)	0.6023	
		Week 16	14	13 (92.9)	7.48 (5.17)	(-3.32, 18.27)	13.87 (7.98)	(-2.79, 30.52)	0.0978	
		Week 20	14	14 (100.0)	4.95 (5.24)	(-5.95, 15.84)	10.21 (8.25)	(-6.96, 27.37)	0.2299	
	Week 26	14	12 (85.7)	3.42 (4.60)	(-6.24, 13.08)	7.98 (7.27)	(-7.29, 23.25)	0.2867		
	SoC	Week 4	10	10 (100.0)	-9.91 (6.00)	(-22.41, 2.60)				
		Week 8	10	10 (100.0)	-4.80 (6.56)	(-18.52, 8.93)				
		Week 12	10	10 (100.0)	-1.89 (6.48)	(-15.40, 11.62)				
		Week 16	10	10 (100.0)	-6.39 (5.92)	(-18.75, 5.97)				
		Week 20	10	10 (100.0)	-5.26 (6.23)	(-18.21, 7.69)				
	Week 26	10	9 (90.0)	-4.56 (5.36)	(-15.82, 6.69)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SIIHA: Change from baseline in QLQ-C30 Cognitive Function (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Cognitive Function (censored)	< 4	Baseline	Peg	21	21 (100.0)	78.57 (18.37)	33.3	83.33	100.0	
			SoC	8	6 (75.0)	86.11 (19.48)	50.0	91.67	100.0	
		Week 4	Peg	21	18 (85.7)	91.67 (11.79)	66.7	100.00	100.0	
			SoC	8	7 (87.5)	85.71 (20.25)	50.0	100.00	100.0	
		Week 8	Peg	21	17 (81.0)	87.25 (15.06)	66.7	100.00	100.0	
			SoC	8	6 (75.0)	83.33 (21.08)	50.0	91.67	100.0	
		Week 12	Peg	21	18 (85.7)	87.04 (15.71)	50.0	91.67	100.0	
			SoC	8	6 (75.0)	83.33 (21.08)	50.0	91.67	100.0	
		Week 16	Peg	21	17 (81.0)	89.22 (13.10)	66.7	100.00	100.0	
			SoC	8	7 (87.5)	78.57 (20.89)	50.0	66.67	100.0	
		Week 20	Peg	21	18 (85.7)	86.11 (20.81)	16.7	91.67	100.0	
			SoC	8	7 (87.5)	83.33 (21.52)	50.0	100.00	100.0	
		Week 26	Peg	21	15 (71.4)	88.89 (15.00)	50.0	100.00	100.0	
			SoC	8	4 (50.0)	79.17 (25.00)	50.0	83.33	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SIIHA: Change from baseline in QLQ-C30 Cognitive Function (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Cognitive Function (censored)	>= 4	Baseline	Peg	14	14 (100.0)	78.57 (23.96)	33.3	83.33	100.0	
			SoC	10	10 (100.0)	88.33 (13.72)	66.7	91.67	100.0	
	Week 4	Peg	14	13 (92.9)	78.21 (22.96)	16.7	83.33	100.0		
		SoC	10	10 (100.0)	76.67 (23.83)	16.7	83.33	100.0		
	Week 8	Peg	14	12 (85.7)	81.94 (24.06)	16.7	83.33	100.0		
		SoC	10	10 (100.0)	80.00 (18.92)	50.0	75.00	100.0		
	Week 12	Peg	14	13 (92.9)	83.33 (23.57)	16.7	83.33	100.0		
		SoC	10	10 (100.0)	83.33 (19.25)	50.0	91.67	100.0		
	Week 16	Peg	14	13 (92.9)	88.46 (20.84)	33.3	100.00	100.0		
		SoC	10	10 (100.0)	78.33 (17.66)	50.0	75.00	100.0		
	Week 20	Peg	14	14 (100.0)	85.71 (23.44)	16.7	100.00	100.0		
		SoC	10	10 (100.0)	80.00 (17.21)	50.0	83.33	100.0		
	Week 26	Peg	14	12 (85.7)	83.33 (18.80)	50.0	91.67	100.0		
		SoC	10	9 (90.0)	81.48 (15.47)	66.7	83.33	100.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QTWC_SIHA: Change from baseline in QLQ-C30 Cognitive Function (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in QLQ-C30 Cognitive Function (censored)	Week 4	Peg	21	18 (85.7)	11.11 (18.96)	-16.7	8.33	50.0	0.94 (0.55) [-0.05, 2.14]
		SoC	8	5 (62.5)	-6.67 (14.91)	-33.3	0.00	0.0	
	Week 8	Peg	21	17 (81.0)	6.86 (21.29)	-33.3	0.00	50.0	0.67 (0.55) [-0.34, 1.83]
		SoC	8	5 (62.5)	-6.67 (9.13)	-16.7	0.00	0.0	
	Week 12	Peg	21	18 (85.7)	6.48 (19.08)	-33.3	0.00	50.0	0.72 (0.55) [-0.28, 1.88]
		SoC	8	5 (62.5)	-6.67 (9.13)	-16.7	0.00	0.0	
	Week 16	Peg	21	17 (81.0)	9.80 (16.73)	-16.7	16.67	50.0	1.26 (0.54) [0.32, 2.46]
		SoC	8	6 (75.0)	-11.11 (13.61)	-33.3	-8.33	0.0	
	Week 20	Peg	21	18 (85.7)	4.63 (25.44)	-66.7	0.00	50.0	0.59 (0.54) [-0.41, 1.73]
		SoC	8	5 (62.5)	-10.00 (14.91)	-33.3	0.00	0.0	
	Week 26	Peg	21	15 (71.4)	7.78 (17.67)	-16.7	0.00	50.0	0.75 (0.69) [-0.51, 2.24]
		SoC	8	3 (37.5)	-5.56 (9.62)	-16.7	0.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QTCW_SIIHA: Change from baseline in QLQ-C30 Cognitive Function (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline \geq 4 in QLQ-C30 Cognitive Function (censored)	Week 4	Peg	14	13 (92.9)	1.28 (20.93)	-50.0	0.00	33.3	0.66 (0.46) [-0.17, 1.63]
		SoC	10	10 (100.0)	-11.67 (15.81)	-50.0	-8.33	0.0	
	Week 8	Peg	14	12 (85.7)	5.56 (27.83)	-50.0	0.00	50.0	0.59 (0.46) [-0.26, 1.57]
		SoC	10	10 (100.0)	-8.33 (14.16)	-33.3	-8.33	16.7	
	Week 12	Peg	14	13 (92.9)	5.13 (26.69)	-50.0	0.00	50.0	0.43 (0.45) [-0.41, 1.36]
		SoC	10	10 (100.0)	-5.00 (15.81)	-33.3	0.00	16.7	
	Week 16	Peg	14	13 (92.9)	10.26 (26.82)	-33.3	0.00	66.7	0.88 (0.47) [0.05, 1.89]
		SoC	10	10 (100.0)	-10.00 (14.05)	-33.3	-16.67	16.7	
	Week 20	Peg	14	14 (100.0)	7.14 (25.91)	-50.0	0.00	50.0	0.68 (0.45) [-0.13, 1.64]
		SoC	10	10 (100.0)	-8.33 (14.16)	-33.3	-8.33	16.7	
	Week 26	Peg	14	12 (85.7)	6.94 (21.86)	-16.7	0.00	50.0	0.81 (0.49) [-0.05, 1.88]
		SoC	10	9 (90.0)	-9.26 (14.70)	-33.3	-16.67	16.7	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SICC: Change from baseline in QLQ-C30 Social Functioning (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.4385

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SICC: Change from baseline in QLQ-C30 Social Functioning (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 60	Peg	Week 4	29	26 (89.7)	13.56 (4.93)	(3.54, 23.59)	9.64 (9.14)	(-8.96, 28.24)	0.2994	
		Week 8	29	24 (82.8)	16.62 (4.80)	(6.84, 26.41)	10.92 (8.66)	(-6.75, 28.58)	0.2170	
		Week 12	29	26 (89.7)	15.31 (5.12)	(4.90, 25.72)	7.54 (9.49)	(-11.77, 26.84)	0.4327	
		Week 16	29	25 (86.2)	19.52 (5.05)	(9.24, 29.80)	11.88 (8.92)	(-6.26, 30.02)	0.1920	
		Week 20	29	27 (93.1)	18.52 (4.42)	(9.54, 27.51)	12.47 (8.31)	(-4.42, 29.36)	0.1427	
		Week 26	29	23 (79.3)	18.44 (4.68)	(8.87, 28.01)	14.19 (8.72)	(-3.65, 32.04)	0.1145	
	SoC	Week 4	13	11 (84.6)	3.92 (7.60)	(-11.54, 19.39)				
		Week 8	13	11 (84.6)	5.71 (7.11)	(-8.79, 20.20)				
		Week 12	13	11 (84.6)	7.77 (7.89)	(-8.28, 23.82)				
		Week 16	13	12 (92.3)	7.64 (7.29)	(-7.19, 22.48)				
		Week 20	13	11 (84.6)	6.05 (6.97)	(-8.12, 20.22)				
		Week 26	13	10 (76.9)	4.25 (7.24)	(-10.56, 19.05)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SICC: Change from baseline in QLQ-C30 Social Functioning (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	-11.23 (12.86)	(-44.28, 21.82)	-6.28 (22.74)	(-64.73, 52.17)	0.7935	
		Week 8	6	5 (83.3)	-4.17 (9.33)	(-28.16, 19.82)	11.11 (16.50)	(-31.32, 53.54)	0.5307	
		Week 12	6	5 (83.3)	-12.26 (8.46)	(-34.00, 9.48)	7.00 (14.96)	(-31.44, 45.45)	0.6592	
		Week 16	6	5 (83.3)	-8.31 (8.77)	(-30.87, 14.24)	13.29 (14.09)	(-22.93, 49.52)	0.3888	
		Week 20	6	5 (83.3)	6.66 (11.74)	(-23.51, 36.82)	30.87 (18.85)	(-17.58, 79.32)	0.1624	
	Week 26	6	4 (66.7)	-12.50 (5.97)	(-38.20, 13.20)	-16.67 (11.79)	(-67.37, 34.04)	0.2929		
	SoC	Week 4	5	4 (80.0)	-4.95 (15.38)	(-44.49, 34.59)				
		Week 8	5	4 (80.0)	-15.28 (11.17)	(-43.98, 13.42)				
		Week 12	5	4 (80.0)	-19.26 (10.12)	(-45.27, 6.74)				
		Week 16	5	4 (80.0)	-21.61 (10.39)	(-48.32, 5.11)				
		Week 20	5	4 (80.0)	-24.21 (13.90)	(-59.95, 11.52)				
Week 26	5	2 (40.0)	4.17 (10.25)	(-39.95, 48.28)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SIHC: Change from baseline in QLQ-C30 Social Functioning (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Social Functioning (censored)	< 60	Baseline	Peg	29	29 (100.0)	66.09 (27.99)	16.7	66.67	100.0	
			SoC	13	12 (92.3)	65.28 (36.56)	0.0	66.67	100.0	
		Week 4	Peg	29	26 (89.7)	81.41 (24.64)	0.0	100.00	100.0	
			SoC	13	12 (92.3)	73.61 (32.92)	0.0	83.33	100.0	
		Week 8	Peg	29	24 (82.8)	84.72 (20.80)	16.7	91.67	100.0	
			SoC	13	11 (84.6)	72.73 (33.56)	0.0	83.33	100.0	
		Week 12	Peg	29	26 (89.7)	84.62 (25.35)	0.0	100.00	100.0	
			SoC	13	11 (84.6)	77.27 (28.16)	33.3	100.00	100.0	
		Week 16	Peg	29	25 (86.2)	87.33 (22.19)	33.3	100.00	100.0	
			SoC	13	12 (92.3)	73.61 (32.92)	0.0	83.33	100.0	
		Week 20	Peg	29	27 (93.1)	87.04 (21.35)	16.7	100.00	100.0	
			SoC	13	12 (92.3)	76.39 (27.02)	33.3	83.33	100.0	
		Week 26	Peg	29	23 (79.3)	86.23 (21.11)	33.3	100.00	100.0	
			SoC	13	10 (76.9)	71.67 (27.27)	33.3	66.67	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SIHC: Change from baseline in QLQ-C30 Social Functioning (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Social Functioning (censored)	>= 60	Baseline	Peg	6	6 (100.0)	86.11 (26.70)	33.3	100.00	100.0	
			SoC	5	4 (80.0)	79.17 (15.96)	66.7	75.00	100.0	
	Week 4	Peg	6	5 (83.3)	93.33 (14.91)	66.7	100.00	100.0		
		SoC	5	5 (100.0)	70.00 (41.50)	0.0	83.33	100.0		
	Week 8	Peg	6	5 (83.3)	96.67 (7.45)	83.3	100.00	100.0		
		SoC	5	5 (100.0)	66.67 (35.36)	16.7	66.67	100.0		
	Week 12	Peg	6	5 (83.3)	90.00 (14.91)	66.7	100.00	100.0		
		SoC	5	5 (100.0)	63.33 (38.01)	16.7	66.67	100.0		
	Week 16	Peg	6	5 (83.3)	76.67 (32.49)	33.3	100.00	100.0		
		SoC	5	5 (100.0)	63.33 (38.01)	16.7	66.67	100.0		
	Week 20	Peg	6	5 (83.3)	90.00 (14.91)	66.7	100.00	100.0		
		SoC	5	5 (100.0)	63.33 (38.01)	16.7	66.67	100.0		
	Week 26	Peg	6	4 (66.7)	83.33 (19.25)	66.7	83.33	100.0		
		SoC	5	3 (60.0)	88.89 (19.25)	66.7	100.00	100.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SIHC: Change from baseline in QLQ-C30 Social Functioning (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in QLQ-C30 Social Functioning (censored)	Week 4	Peg	29	26 (89.7)	14.74 (32.77)	-100.0	16.67	66.7	0.43 (0.37) [-0.28, 1.20]
		SoC	13	11 (84.6)	1.52 (21.67)	-50.0	0.00	33.3	
	Week 8	Peg	29	24 (82.8)	18.06 (31.82)	-83.3	16.67	66.7	0.49 (0.38) [-0.22, 1.28]
		SoC	13	11 (84.6)	3.03 (24.52)	-50.0	0.00	33.3	
	Week 12	Peg	29	26 (89.7)	15.38 (36.19)	-100.0	16.67	66.7	0.23 (0.37) [-0.49, 0.98]
		SoC	13	11 (84.6)	7.58 (25.13)	-50.0	0.00	33.3	
	Week 16	Peg	29	25 (86.2)	19.33 (32.16)	-66.7	16.67	66.7	0.33 (0.36) [-0.37, 1.07]
		SoC	13	12 (92.3)	8.33 (34.45)	-50.0	0.00	83.3	
	Week 20	Peg	29	27 (93.1)	19.14 (33.87)	-83.3	16.67	66.7	0.44 (0.37) [-0.26, 1.20]
		SoC	13	11 (84.6)	4.55 (27.98)	-50.0	0.00	33.3	
	Week 26	Peg	29	23 (79.3)	18.12 (31.35)	-66.7	16.67	66.7	0.42 (0.40) [-0.33, 1.23]
		SoC	13	10 (76.9)	5.00 (29.45)	-50.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SIHC: Change from baseline in QLQ-C30 Social Functioning (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in QLQ-C30 Social Functioning (censored)	Week 4	Peg	6	5 (83.3)	-3.33 (7.45)	-16.7	0.00	0.0	0.49 (0.81) [-0.94, 2.30]
		SoC	5	4 (80.0)	-16.67 (36.00)	-66.7	-8.33	16.7	
	Week 8	Peg	6	5 (83.3)	0.00 (11.79)	-16.7	0.00	16.7	1.13 (0.90) [-0.19, 3.32]
		SoC	5	4 (80.0)	-20.83 (20.97)	-50.0	-16.67	0.0	
	Week 12	Peg	6	5 (83.3)	-6.67 (14.91)	-33.3	0.00	0.0	0.90 (0.86) [-0.45, 2.95]
		SoC	5	4 (80.0)	-25.00 (21.52)	-50.0	-25.00	0.0	
	Week 16	Peg	6	5 (83.3)	-6.67 (14.91)	-33.3	0.00	0.0	0.90 (0.86) [-0.45, 2.95]
		SoC	5	4 (80.0)	-25.00 (21.52)	-50.0	-25.00	0.0	
	Week 20	Peg	6	5 (83.3)	6.67 (25.28)	-16.7	0.00	50.0	1.19 (0.90) [-0.14, 3.41]
		SoC	5	4 (80.0)	-25.00 (21.52)	-50.0	-25.00	0.0	
	Week 26	Peg	6	4 (66.7)	-12.50 (15.96)	-33.3	-8.33	0.0	-0.22 (1.23) [-2.82, 2.01]
		SoC	5	2 (40.0)	-8.33 (11.79)	-16.7	-8.33	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SICD: Change from baseline in QLQ-C30 Social Functioning (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.3617

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SICD: Change from baseline in QLQ-C30 Social Functioning (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Male	Peg	Week 4	19	16 (84.2)	-0.52 (6.34)	(-13.75, 12.71)	-0.57 (11.19)	(-23.92, 22.78)	0.9597	
		Week 8	19	15 (78.9)	0.21 (5.93)	(-12.19, 12.62)	0.55 (10.25)	(-20.90, 22.00)	0.9576	
		Week 12	19	17 (89.5)	-4.85 (6.81)	(-19.02, 9.32)	-4.94 (12.40)	(-30.73, 20.85)	0.6944	
		Week 16	19	16 (84.2)	3.44 (6.96)	(-11.03, 17.90)	1.13 (11.88)	(-23.58, 25.84)	0.9253	
		Week 20	19	17 (89.5)	2.69 (6.05)	(-9.89, 15.28)	8.26 (10.81)	(-14.22, 30.75)	0.4532	
		Week 26	19	14 (73.7)	-0.30 (5.82)	(-12.63, 12.04)	1.79 (11.47)	(-22.53, 26.11)	0.8782	
	SoC	Week 4	10	8 (80.0)	0.05 (9.03)	(-18.79, 18.89)				
		Week 8	10	8 (80.0)	-0.34 (8.19)	(-17.49, 16.81)				
		Week 12	10	8 (80.0)	0.09 (10.09)	(-20.88, 21.06)				
		Week 16	10	9 (90.0)	2.31 (9.41)	(-17.25, 21.87)				
		Week 20	10	8 (80.0)	-5.57 (8.88)	(-24.03, 12.89)				
		Week 26	10	6 (60.0)	-2.08 (9.30)	(-21.80, 17.63)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SICD: Change from baseline in QLQ-C30 Social Functioning (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Female	Peg	Week 4	16	15 (93.8)	25.64 (6.43)	(12.12, 39.16)	39.97 (14.29)	(9.95, 70.00)	0.0119	*
		Week 8	16	14 (87.5)	29.94 (6.01)	(17.26, 42.62)	37.83 (13.08)	(10.23, 65.43)	0.0101	*
		Week 12	16	14 (87.5)	33.27 (5.58)	(21.51, 45.04)	40.43 (12.14)	(14.82, 66.05)	0.0040	*
		Week 16	16	14 (87.5)	31.06 (6.77)	(16.76, 45.35)	42.51 (14.75)	(11.40, 73.62)	0.0103	*
		Week 20	16	15 (93.8)	33.49 (5.36)	(22.24, 44.75)	39.21 (11.90)	(14.21, 64.22)	0.0040	*
		Week 26	16	13 (81.3)	30.49 (5.87)	(17.97, 43.01)	30.82 (12.99)	(3.13, 58.51)	0.0315	*
	SoC	Week 4	8	7 (87.5)	-14.33 (11.69)	(-38.90, 10.24)				
		Week 8	8	7 (87.5)	-7.89 (10.38)	(-29.80, 14.02)				
		Week 12	8	7 (87.5)	-7.16 (9.64)	(-27.49, 13.17)				
		Week 16	8	7 (87.5)	-11.45 (11.70)	(-36.15, 13.24)				
		Week 20	8	7 (87.5)	-5.72 (9.74)	(-26.18, 14.74)				
		Week 26	8	6 (75.0)	-0.33 (10.55)	(-22.82, 22.15)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SIHD: Change from baseline in QLQ-C30 Social Functioning (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Social Functioning (censored)	Male	Baseline	Peg	19	19 (100.0)	81.58 (20.71)	33.3	83.33	100.0	
			SoC	10	9 (90.0)	64.81 (31.67)	16.7	66.67	100.0	
		Week 4	Peg	19	16 (84.2)	82.29 (26.85)	0.0	100.00	100.0	
			SoC	10	9 (90.0)	79.63 (23.24)	33.3	83.33	100.0	
		Week 8	Peg	19	15 (78.9)	82.22 (24.77)	16.7	100.00	100.0	
			SoC	10	9 (90.0)	77.78 (25.00)	33.3	83.33	100.0	
		Week 12	Peg	19	17 (89.5)	78.43 (28.73)	0.0	83.33	100.0	
			SoC	10	9 (90.0)	77.78 (28.87)	33.3	100.00	100.0	
		Week 16	Peg	19	16 (84.2)	80.21 (27.36)	33.3	100.00	100.0	
			SoC	10	10 (100.0)	76.67 (27.44)	33.3	83.33	100.0	
		Week 20	Peg	19	17 (89.5)	82.35 (23.91)	16.7	100.00	100.0	
			SoC	10	9 (90.0)	74.07 (27.78)	33.3	66.67	100.0	
		Week 26	Peg	19	14 (73.7)	83.33 (20.67)	33.3	91.67	100.0	
			SoC	10	7 (70.0)	76.19 (25.20)	33.3	66.67	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SIHD: Change from baseline in QLQ-C30 Social Functioning (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Social Functioning (censored)	Female	Baseline	Peg	16	16 (100.0)	55.21 (30.26)	16.7	41.67	100.0	
			SoC	8	7 (87.5)	73.81 (35.82)	0.0	83.33	100.0	
		Week 4	Peg	16	15 (93.8)	84.44 (20.38)	50.0	100.00	100.0	
			SoC	8	8 (100.0)	64.58 (44.04)	0.0	83.33	100.0	
		Week 8	Peg	16	14 (87.5)	91.67 (10.84)	66.7	100.00	100.0	
			SoC	8	7 (87.5)	61.90 (41.63)	0.0	66.67	100.0	
		Week 12	Peg	16	14 (87.5)	94.05 (12.42)	66.7	100.00	100.0	
			SoC	8	7 (87.5)	66.67 (34.69)	16.7	66.67	100.0	
		Week 16	Peg	16	14 (87.5)	91.67 (18.20)	50.0	100.00	100.0	
			SoC	8	7 (87.5)	61.90 (41.63)	0.0	66.67	100.0	
		Week 20	Peg	16	15 (93.8)	93.33 (13.80)	66.7	100.00	100.0	
			SoC	8	8 (100.0)	70.83 (34.21)	16.7	83.33	100.0	
		Week 26	Peg	16	13 (81.3)	88.46 (20.84)	33.3	100.00	100.0	
			SoC	8	6 (75.0)	75.00 (29.34)	33.3	83.33	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SIHD: Change from baseline in QLQ-C30 Social Functioning (censored) by sex
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Male in QLQ-C30 Social Functioning (censored)	Week 4	Peg	19	16 (84.2)	-2.08 (30.96)	-100.0	0.00	33.3	-0.29 (0.46) [-1.23, 0.58]
		SoC	10	8 (80.0)	6.25 (17.68)	-16.7	0.00	33.3	
	Week 8	Peg	19	15 (78.9)	-1.11 (26.33)	-83.3	0.00	33.3	-0.21 (0.46) [-1.15, 0.68]
		SoC	10	8 (80.0)	4.17 (19.42)	-16.7	0.00	33.3	
	Week 12	Peg	19	17 (89.5)	-6.86 (30.08)	-100.0	0.00	33.3	-0.38 (0.45) [-1.32, 0.47]
		SoC	10	8 (80.0)	4.17 (21.36)	-33.3	0.00	33.3	
	Week 16	Peg	19	16 (84.2)	0.00 (27.89)	-66.7	0.00	33.3	-0.29 (0.44) [-1.18, 0.55]
		SoC	10	9 (90.0)	9.26 (36.43)	-33.3	0.00	83.3	
	Week 20	Peg	19	17 (89.5)	0.98 (30.32)	-83.3	0.00	50.0	0.03 (0.45) [-0.85, 0.92]
		SoC	10	8 (80.0)	0.00 (25.20)	-33.3	0.00	33.3	
	Week 26	Peg	19	14 (73.7)	-3.57 (26.29)	-66.7	0.00	33.3	-0.34 (0.52) [-1.41, 0.65]
		SoC	10	6 (60.0)	5.56 (25.09)	-33.3	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SIHD: Change from baseline in QLQ-C30 Social Functioning (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Female in QLQ-C30 Social Functioning (censored)	Week 4	Peg	16	15 (93.8)	26.67 (23.40)	0.0	16.67	66.7	1.52 (0.55)	[0.61, 2.78]
		SoC	8	7 (87.5)	-14.29 (31.07)	-66.7	0.00	16.7		
	Week 8	Peg	16	14 (87.5)	32.14 (23.99)	0.0	33.33	66.7	1.62 (0.57)	[0.70, 2.94]
		SoC	8	7 (87.5)	-11.90 (29.99)	-50.0	0.00	33.3		
	Week 12	Peg	16	14 (87.5)	34.52 (24.86)	0.0	33.33	66.7	1.42 (0.55)	[0.50, 2.67]
		SoC	8	7 (87.5)	-7.14 (34.50)	-50.0	0.00	33.3		
	Week 16	Peg	16	14 (87.5)	32.14 (26.53)	0.0	25.00	66.7	1.53 (0.56)	[0.61, 2.82]
		SoC	8	7 (87.5)	-11.90 (29.99)	-50.0	0.00	33.3		
	Week 20	Peg	16	15 (93.8)	35.56 (25.09)	0.0	33.33	66.7	1.45 (0.55)	[0.55, 2.69]
		SoC	8	7 (87.5)	-7.14 (34.50)	-50.0	0.00	33.3		
	Week 26	Peg	16	13 (81.3)	32.05 (25.88)	0.0	33.33	66.7	1.11 (0.57)	[0.14, 2.38]
		SoC	8	6 (75.0)	0.00 (31.62)	-50.0	0.00	33.3		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SICB: Change from baseline in QLQ-C30 Social Functioning (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.4364

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SICB: Change from baseline in QLQ-C30 Social Functioning (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Latin America	Peg	Week 4	12	11 (91.7)	19.93 (5.92)	(6.53, 33.33)	15.24 (15.92)	(-20.79, 51.26)	0.3636	
		Week 8	12	10 (83.3)	20.39 (5.64)	(7.38, 33.39)	14.00 (14.46)	(-19.34, 47.34)	0.3614	
		Week 12	12	11 (91.7)	21.06 (6.30)	(6.80, 35.33)	21.29 (16.95)	(-17.05, 59.63)	0.2406	
		Week 16	12	10 (83.3)	23.62 (6.76)	(8.04, 39.21)	16.75 (17.33)	(-23.21, 56.71)	0.3621	
		Week 20	12	10 (83.3)	20.11 (6.84)	(4.34, 35.88)	20.68 (17.77)	(-20.31, 61.66)	0.2782	
		Week 26	12	9 (75.0)	23.31 (6.29)	(8.43, 38.18)	22.92 (15.61)	(-13.99, 59.84)	0.1855	
	SoC	Week 4	2	2 (100.0)	4.69 (14.57)	(-28.27, 37.65)				
		Week 8	2	2 (100.0)	6.39 (13.08)	(-23.78, 36.56)				
		Week 12	2	2 (100.0)	-0.23 (15.51)	(-35.31, 34.85)				
		Week 16	2	2 (100.0)	6.88 (15.68)	(-29.28, 43.04)				
		Week 20	2	2 (100.0)	-0.56 (16.04)	(-37.56, 36.43)				
		Week 26	2	2 (100.0)	0.38 (14.03)	(-32.80, 33.56)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SICB: Change from baseline in QLQ-C30 Social Functioning (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Asia Pacific Peg		Week 4	23	20 (87.0)	6.25 (6.16)	(-6.35, 18.86)	9.05 (10.12)	(-11.64, 29.74)	0.3783	
		Week 8	23	19 (82.6)	11.46 (5.92)	(-0.68, 23.59)	15.38 (9.59)	(-4.26, 35.02)	0.1199	
		Week 12	23	20 (87.0)	8.14 (6.45)	(-5.05, 21.32)	11.76 (10.58)	(-9.88, 33.41)	0.2756	
		Week 16	23	20 (87.0)	10.86 (6.51)	(-2.44, 24.15)	12.08 (10.33)	(-9.03, 33.18)	0.2518	
		Week 20	23	22 (95.7)	15.45 (5.37)	(4.50, 26.40)	18.49 (9.06)	(0.00, 36.98)	0.0499	*
		Week 26	23	18 (78.3)	9.18 (5.41)	(-1.99, 20.35)	7.37 (9.62)	(-12.48, 27.23)	0.4509	
SoC		Week 4	16	13 (81.3)	-2.80 (7.78)	(-18.71, 13.11)				
		Week 8	16	13 (81.3)	-3.92 (7.23)	(-18.74, 10.90)				
		Week 12	16	13 (81.3)	-3.62 (8.14)	(-20.27, 13.03)				
		Week 16	16	14 (87.5)	-1.22 (7.82)	(-17.20, 14.76)				
		Week 20	16	13 (81.3)	-3.04 (7.11)	(-17.54, 11.46)				
		Week 26	16	10 (62.5)	1.81 (7.77)	(-14.23, 17.85)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SIHB: Change from baseline in QLQ-C30 Social Functioning (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Social Functioning (censored)	Latin America	Baseline	Peg	12	12 (100.0)	68.06 (29.69)	16.7	66.67	100.0	
			SoC	2	2 (100.0)	50.00 (23.57)	33.3	50.00	66.7	
		Week 4	Peg	12	11 (91.7)	90.91 (17.26)	50.0	100.00	100.0	
			SoC	2	2 (100.0)	66.67 (47.14)	33.3	66.67	100.0	
		Week 8	Peg	12	10 (83.3)	88.33 (15.81)	66.7	100.00	100.0	
			SoC	2	2 (100.0)	66.67 (47.14)	33.3	66.67	100.0	
		Week 12	Peg	12	11 (91.7)	90.91 (13.67)	66.7	100.00	100.0	
			SoC	2	2 (100.0)	66.67 (47.14)	33.3	66.67	100.0	
		Week 16	Peg	12	10 (83.3)	91.67 (18.00)	50.0	100.00	100.0	
			SoC	2	2 (100.0)	66.67 (47.14)	33.3	66.67	100.0	
		Week 20	Peg	12	10 (83.3)	90.00 (16.10)	66.7	100.00	100.0	
			SoC	2	2 (100.0)	66.67 (47.14)	33.3	66.67	100.0	
		Week 26	Peg	12	9 (75.0)	94.44 (11.79)	66.7	100.00	100.0	
			SoC	2	2 (100.0)	66.67 (47.14)	33.3	66.67	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SIHB: Change from baseline in QLQ-C30 Social Functioning (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Social Functioning (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	70.29 (28.41)	16.7	66.67	100.0	
			SoC	16	14 (87.5)	71.43 (33.61)	0.0	75.00	100.0	
		Week 4	Peg	23	20 (87.0)	79.17 (25.86)	0.0	83.33	100.0	
			SoC	16	15 (93.8)	73.33 (34.39)	0.0	83.33	100.0	
		Week 8	Peg	23	19 (82.6)	85.96 (21.70)	16.7	100.00	100.0	
			SoC	16	14 (87.5)	71.43 (32.96)	0.0	75.00	100.0	
		Week 12	Peg	23	20 (87.0)	82.50 (27.82)	0.0	100.00	100.0	
			SoC	16	14 (87.5)	73.81 (30.46)	16.7	83.33	100.0	
		Week 16	Peg	23	20 (87.0)	82.50 (26.20)	33.3	100.00	100.0	
			SoC	16	15 (93.8)	71.11 (33.61)	0.0	66.67	100.0	
		Week 20	Peg	23	22 (95.7)	86.36 (22.20)	16.7	100.00	100.0	
			SoC	16	15 (93.8)	73.33 (29.41)	16.7	66.67	100.0	
		Week 26	Peg	23	18 (78.3)	81.48 (22.79)	33.3	91.67	100.0	
			SoC	16	11 (68.8)	77.27 (23.89)	33.3	66.67	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SIHB: Change from baseline in QLQ-C30 Social Functioning (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline in QLQ-C30 Social Functioning (censored)	Latin America	Week 4	Peg	12	11 (91.7)	18.18 (22.92)	0.0	16.67	66.7	0.06	(0.85) [-1.62, 1.77]
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3		
		Week 8	Peg	12	10 (83.3)	18.33 (21.44)	0.0	8.33	50.0	0.07	(0.87) [-1.64, 1.82]
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3		
		Week 12	Peg	12	11 (91.7)	18.18 (29.30)	-33.3	16.67	66.7	0.05	(0.85) [-1.63, 1.75]
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3		
		Week 16	Peg	12	10 (83.3)	21.67 (23.64)	0.0	16.67	66.7	0.20	(0.87) [-1.48, 1.97]
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3		
		Week 20	Peg	12	10 (83.3)	16.67 (29.40)	-33.3	8.33	66.7		NE (NE) [NE, NE]
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3		
		Week 26	Peg	12	9 (75.0)	20.37 (27.36)	-16.7	16.67	66.7	0.13	(0.89) [-1.61, 1.93]
			SoC	2	2 (100.0)	16.67 (23.57)	0.0	16.67	33.3		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SIHb: Change from baseline in QLQ-C30 Social Functioning (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in QLQ-C30 Social Functioning (censored)	Asia Pacific	Week 4	Peg	23	20 (87.0)	8.33 (34.41)	-100.0	8.33	66.7	0.46 (0.37) [-0.24, 1.23]
			SoC	16	13 (81.3)	-6.41 (25.94)	-66.7	0.00	33.3	
		Week 8	Peg	23	19 (82.6)	13.16 (34.06)	-83.3	16.67	66.7	0.62 (0.38) [-0.09, 1.42]
			SoC	16	13 (81.3)	-6.41 (25.04)	-50.0	0.00	33.3	
		Week 12	Peg	23	20 (87.0)	8.33 (37.27)	-100.0	0.00	66.7	0.35 (0.37) [-0.36, 1.11]
			SoC	16	13 (81.3)	-3.85 (28.18)	-50.0	0.00	33.3	
		Week 16	Peg	23	20 (87.0)	11.67 (34.67)	-66.7	8.33	66.7	0.39 (0.36) [-0.30, 1.14]
			SoC	16	14 (87.5)	-2.38 (35.72)	-50.0	0.00	83.3	
		Week 20	Peg	23	22 (95.7)	17.42 (34.69)	-83.3	16.67	66.7	0.71 (0.37) [0.02, 1.49]
			SoC	16	13 (81.3)	-6.41 (29.30)	-50.0	0.00	33.3	
		Week 26	Peg	23	18 (78.3)	10.19 (33.40)	-66.7	8.33	66.7	0.31 (0.41) [-0.48, 1.15]
			SoC	16	10 (62.5)	0.00 (28.33)	-50.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SICA: Change from baseline in QLQ-C30 Social Functioning (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.5403

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SICA: Change from baseline in QLQ-C30 Social Functioning (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 4	21	18 (85.7)	13.77 (3.61)	(6.25, 21.29)	3.33 (7.77)	(-12.88, 19.54)	0.6726	
		Week 8	21	17 (81.0)	18.40 (3.76)	(10.52, 26.27)	10.95 (7.91)	(-5.62, 27.51)	0.1827	
		Week 12	21	18 (85.7)	11.11 (5.10)	(0.46, 21.75)	7.76 (11.00)	(-15.19, 30.71)	0.4888	
		Week 16	21	17 (81.0)	18.31 (5.79)	(6.23, 30.40)	3.53 (11.45)	(-20.36, 27.42)	0.7613	
		Week 20	21	18 (85.7)	18.93 (4.31)	(9.94, 27.93)	13.76 (9.25)	(-5.54, 33.06)	0.1526	
		Week 26	21	15 (71.4)	16.87 (4.35)	(7.61, 26.13)	1.21 (10.88)	(-21.97, 24.40)	0.9126	
	SoC	Week 4	8	5 (62.5)	10.43 (6.87)	(-3.89, 24.76)				
		Week 8	8	5 (62.5)	7.45 (6.95)	(-7.10, 22.00)				
		Week 12	8	5 (62.5)	3.35 (9.72)	(-16.93, 23.62)				
		Week 16	8	6 (75.0)	14.78 (9.81)	(-5.68, 35.25)				
		Week 20	8	5 (62.5)	5.17 (8.18)	(-11.90, 22.24)				
		Week 26	8	3 (37.5)	15.65 (9.89)	(-5.42, 36.73)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SICA: Change from baseline in QLQ-C30 Social Functioning (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	8.92 (8.93)	(-9.71, 27.55)	18.85 (13.58)	(-9.48, 47.17)	0.1804	
		Week 8	14	12 (85.7)	10.39 (8.33)	(-7.03, 27.82)	19.53 (12.36)	(-6.33, 45.40)	0.1304	
		Week 12	14	13 (92.9)	13.89 (8.41)	(-3.64, 31.42)	18.61 (12.75)	(-7.98, 45.20)	0.1598	
		Week 16	14	13 (92.9)	12.65 (8.00)	(-4.03, 29.33)	24.10 (12.13)	(-1.20, 49.40)	0.0608	
		Week 20	14	14 (100.0)	14.29 (7.46)	(-1.22, 29.81)	20.97 (11.57)	(-3.09, 45.03)	0.0842	
		Week 26	14	12 (85.7)	10.79 (7.23)	(-4.39, 25.98)	14.07 (11.04)	(-9.13, 37.27)	0.2187	
	SoC	Week 4	10	10 (100.0)	-9.93 (10.19)	(-31.19, 11.33)				
		Week 8	10	10 (100.0)	-9.14 (9.12)	(-28.23, 9.95)				
		Week 12	10	10 (100.0)	-4.72 (9.58)	(-24.71, 15.27)				
		Week 16	10	10 (100.0)	-11.45 (9.12)	(-30.47, 7.57)				
		Week 20	10	10 (100.0)	-6.68 (8.83)	(-25.04, 11.68)				
		Week 26	10	9 (90.0)	-3.28 (8.35)	(-20.81, 14.26)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SIHA: Change from baseline in QLQ-C30 Social Functioning (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Social Functioning (censored)	< 4	Baseline	Peg	21	21 (100.0)	68.25 (27.34)	16.7	66.67	100.0	
			SoC	8	6 (75.0)	58.33 (29.34)	16.7	66.67	100.0	
		Week 4	Peg	21	18 (85.7)	86.11 (18.30)	50.0	100.00	100.0	
			SoC	8	7 (87.5)	85.71 (17.82)	66.7	100.00	100.0	
		Week 8	Peg	21	17 (81.0)	89.22 (15.52)	50.0	100.00	100.0	
			SoC	8	6 (75.0)	80.56 (22.15)	50.0	83.33	100.0	
		Week 12	Peg	21	18 (85.7)	83.33 (21.39)	33.3	100.00	100.0	
			SoC	8	6 (75.0)	77.78 (27.22)	33.3	83.33	100.0	
		Week 16	Peg	21	17 (81.0)	84.31 (23.91)	33.3	100.00	100.0	
			SoC	8	7 (87.5)	80.95 (26.23)	33.3	100.00	100.0	
		Week 20	Peg	21	18 (85.7)	87.96 (16.96)	50.0	100.00	100.0	
			SoC	8	7 (87.5)	80.95 (26.23)	33.3	100.00	100.0	
		Week 26	Peg	21	15 (71.4)	85.56 (19.79)	33.3	100.00	100.0	
			SoC	8	4 (50.0)	83.33 (19.25)	66.7	83.33	100.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SIHA: Change from baseline in QLQ-C30 Social Functioning (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
QLQ-C30 Social Functioning (censored)	>= 4	Baseline	Peg	14	14 (100.0)	71.43 (30.96)	33.3	83.33	100.0	
			SoC	10	10 (100.0)	75.00 (34.47)	0.0	91.67	100.0	
	Week 4	Peg	14	13 (92.9)	79.49 (29.78)	0.0	100.00	100.0		
		SoC	10	10 (100.0)	63.33 (40.67)	0.0	75.00	100.0		
	Week 8	Peg	14	12 (85.7)	83.33 (24.62)	16.7	91.67	100.0		
		SoC	10	10 (100.0)	65.00 (38.05)	0.0	75.00	100.0		
	Week 12	Peg	14	13 (92.9)	88.46 (27.54)	0.0	100.00	100.0		
		SoC	10	10 (100.0)	70.00 (34.07)	16.7	83.33	100.0		
	Week 16	Peg	14	13 (92.9)	87.18 (24.68)	33.3	100.00	100.0		
		SoC	10	10 (100.0)	63.33 (37.52)	0.0	66.67	100.0		
	Week 20	Peg	14	14 (100.0)	86.90 (24.62)	16.7	100.00	100.0		
		SoC	10	10 (100.0)	66.67 (32.39)	16.7	66.67	100.0		
	Week 26	Peg	14	12 (85.7)	86.11 (22.29)	33.3	100.00	100.0		
		SoC	10	9 (90.0)	72.22 (28.87)	33.3	66.67	100.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SIHA: Change from baseline in QLQ-C30 Social Functioning (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in QLQ-C30 Social Functioning (censored)	Week 4	Peg	21	18 (85.7)	12.96 (21.05)	-33.3	8.33	50.0	-0.02 (0.53) [-1.07, 1.03]
		SoC	8	5 (62.5)	13.33 (18.26)	0.0	0.00	33.3	
	Week 8	Peg	21	17 (81.0)	17.65 (22.42)	-16.7	16.67	66.7	0.33 (0.54) [-0.70, 1.43]
		SoC	8	5 (62.5)	10.00 (22.36)	-16.7	0.00	33.3	
	Week 12	Peg	21	18 (85.7)	10.19 (26.28)	-33.3	8.33	50.0	0.13 (0.53) [-0.91, 1.19]
		SoC	8	5 (62.5)	6.67 (27.89)	-33.3	0.00	33.3	
	Week 16	Peg	21	17 (81.0)	16.67 (25.00)	-33.3	16.67	66.7	-0.09 (0.50) [-1.09, 0.89]
		SoC	8	6 (75.0)	19.44 (40.02)	-33.3	16.67	83.3	
	Week 20	Peg	21	18 (85.7)	18.52 (24.18)	-33.3	25.00	50.0	0.46 (0.54) [-0.55, 1.57]
		SoC	8	5 (62.5)	6.67 (27.89)	-33.3	0.00	33.3	
	Week 26	Peg	21	15 (71.4)	15.56 (21.33)	-16.7	16.67	50.0	-0.30 (0.68) [-1.69, 1.00]
		SoC	8	3 (37.5)	22.22 (19.25)	0.0	33.33	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QSWC_SIHA: Change from baseline in QLQ-C30 Social Functioning (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in QLQ-C30 Social Functioning (censored)	Week 4	Peg	14	13 (92.9)	10.26 (41.69)	-100.0	16.67	66.7	0.59 (0.45) [-0.24, 1.55]
		SoC	10	10 (100.0)	-11.67 (26.12)	-66.7	0.00	16.7	
	Week 8	Peg	14	12 (85.7)	11.11 (39.14)	-83.3	8.33	66.7	0.61 (0.46) [-0.24, 1.59]
		SoC	10	10 (100.0)	-10.00 (25.09)	-50.0	0.00	33.3	
	Week 12	Peg	14	13 (92.9)	14.10 (44.54)	-100.0	0.00	66.7	0.48 (0.45) [-0.36, 1.42]
		SoC	10	10 (100.0)	-5.00 (28.38)	-50.0	0.00	33.3	
	Week 16	Peg	14	13 (92.9)	12.82 (39.18)	-66.7	0.00	66.7	0.69 (0.46) [-0.14, 1.67]
		SoC	10	10 (100.0)	-11.67 (26.12)	-50.0	0.00	33.3	
	Week 20	Peg	14	14 (100.0)	15.48 (42.09)	-83.3	0.00	66.7	0.61 (0.45) [-0.20, 1.56]
		SoC	10	10 (100.0)	-8.33 (29.66)	-50.0	0.00	33.3	
	Week 26	Peg	14	12 (85.7)	11.11 (41.64)	-66.7	0.00	66.7	0.39 (0.47) [-0.49, 1.37]
		SoC	10	9 (90.0)	-3.70 (27.36)	-50.0	0.00	33.3	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1QGWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								0.5704

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QGWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by age ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	19 (65.5)	8.00	(4.14, 16.14)	1.49	(0.59, 3.74)	0.3974	
	SoC	13	6 (46.2)	12.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QGWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by age ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	4 (66.7)	8.43	(4.14, NE)	2.77	(0.30, 25.44)	0.3666	
	SoC	5	1 (20.0)	NE	(3.86, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QGWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.1522

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QGWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by sex ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	12 (63.2)	4.57	(4.14, NE)	2.69	(0.75, 9.58)	0.1277	
	SoC	10	3 (30.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QGWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	11 (68.8)	8.14	(4.14, 16.14)	0.60	(0.15, 2.52)	0.4895	
	SoC	8	4 (50.0)	8.14	(3.86, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QGWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QGWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	10 (83.3)	4.14	(4.14, 8.00)	NE	(NE, NE)	NE	
	SoC	2	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QGWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	13 (56.5)	12.14	(4.14, NE)	1.06	(0.42, 2.67)	0.8993	
	SoC	16	7 (43.8)	14.29	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QGWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								0.2414

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QGWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	14 (66.7)	8.14	(4.14, 20.14)	3.10	(0.70, 13.70)	0.1360	
	SoC	8	2 (25.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QGWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	9 (64.3)	8.14	(4.14, NE)	1.02	(0.33, 3.14)	0.9786	
	SoC	10	5 (50.0)	12.14	(3.86, 12.14)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QYWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored) by age ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QYWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored) by age ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	17 (58.6)	4.14	(4.14, 8.14)	3.56	(1.03, 12.28)	0.0445	*
	SoC	13	3 (23.1)	16.43	(12.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QYWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored) by
 age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	2 (33.3)	10.36	(4.29, 16.43)	NE	(NE, NE)	NE	
	SoC	5	1 (20.0)	NE	(8.29, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QYWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored) by sex ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.7299

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QYWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored) by sex ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	10 (52.6)	4.29	(4.14, 16.43)	4.41	(0.96, 20.30)	0.0567	
	SoC	10	2 (20.0)	NE	(16.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QYWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored) by sex ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	9 (56.3)	4.14	(4.00, 12.00)	2.72	(0.49, 15.06)	0.2512	
	SoC	8	2 (25.0)	12.14	(8.29, 12.14)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QYWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QYWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	7 (58.3)	4.14	(4.14, 4.29)	NE	(NE, NE)	NE	
	SoC	2	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QYWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	12 (52.2)	6.21	(4.14, 16.43)	2.77	(0.88, 8.77)	0.0827	
	SoC	16	4 (25.0)	16.43	(8.29, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QYWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								0.8215

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QYWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	11 (52.4)	4.36	(4.14, 16.43)	3.31	(0.73, 15.08)	0.1212	
	SoC	8	2 (25.0)	NE	(16.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QYWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	8 (57.1)	4.14	(4.14, 12.14)	4.80	(1.00, 23.13)	0.0504	
	SoC	10	2 (20.0)	12.14	(8.29, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by age ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	18 (62.1)	4.14	(NE, NE)	6.25	(1.75, 22.28)	0.0048	*
	SoC	13	3 (23.1)	8.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	1 (16.7)	NE	(4.57, NE)	NE	(NE, NE)	NE	
	SoC	5	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.9632

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	9 (47.4)	4.14	(4.14, 20.43)	6.61	(0.81, 54.02)	0.0780	
	SoC	10	1 (10.0)	NE	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	10 (62.5)	4.14	(3.86, 4.14)	12.65	(1.28, 125.13)	0.0300	*
	SoC	8	2 (25.0)	8.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by region ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	8 (66.7)	4.14	(4.14, 4.57)	NE	(NE, NE)	NE	
	SoC	2	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	11 (47.8)	4.14	(4.14, 8.29)	3.60	(0.98, 13.17)	0.0529	
	SoC	16	3 (18.8)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								0.4186

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	13 (61.9)	4.14	(4.14, 4.57)	9.56	(1.24, 73.93)	0.0306	*
	SoC	8	1 (12.5)	NE	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	6 (42.9)	4.14	(3.86, 4.14)	3.15	(0.62, 16.03)	0.1677	
	SoC	10	2 (20.0)	8.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QEWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)
 by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QEWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)
 by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	17 (58.6)	4.21	(4.14, 8.14)	3.72	(1.23, 11.28)	0.0201	*
	SoC	13	4 (30.8)	16.43	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QEWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)
 by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	2 (33.3)	10.36	(4.57, 16.14)	NE	(NE, NE)	NE	
	SoC	5	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QEWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)
 by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.2405

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QEWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)
 by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	9 (47.4)	4.57	(4.14, 16.14)	10.20	(1.27, 82.07)	0.0290	*
	SoC	10	1 (10.0)	NE	(16.43, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QEWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)
 by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	10 (62.5)	4.29	(4.14, 8.14)	3.07	(0.61, 15.36)	0.1724	
	SoC	8	3 (37.5)	8.29	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QEWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)
 by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QEWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)
 by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	8 (66.7)	4.21	(4.14, 4.57)	NE	(NE, NE)	NE	
	SoC	2	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QEWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)
 by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	11 (47.8)	8.14	(4.14, 16.14)	3.25	(1.03, 10.32)	0.0452	*
	SoC	16	4 (25.0)	NE	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QEWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)
 by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								0.2917

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QEWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)
 by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	13 (61.9)	4.57	(4.14, 8.14)	9.18	(1.17, 71.80)	0.0347	*
	SoC	8	1 (12.5)	NE	(16.43, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QEWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)
 by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Emotional Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	6 (42.9)	4.29	(4.14, 16.14)	2.46	(0.59, 10.25)	0.2149	
	SoC	10	3 (30.0)	NE	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.

95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).

A Cox proportional model was applied with factor treatment.

Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.

No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.

Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	15 (51.7)	4.57	(4.14, 16.14)	3.56	(0.81, 15.61)	0.0927	
	SoC	13	2 (15.4)	NE	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by age
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	2 (33.3)	NE	(4.57, NE)	NE	(NE, NE)	NE	
	SoC	5	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.6705

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	6 (31.6)	16.14	(4.14, NE)	3.53	(0.42, 29.50)	0.2446	
	SoC	10	1 (10.0)	NE	(12.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by sex
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	11 (68.8)	6.29	(4.14, 16.14)	2.08	(0.23, 19.16)	0.5170	
	SoC	8	1 (12.5)	NE	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	7 (58.3)	4.14	(4.14, 4.57)	NE	(NE, NE)	NE	
	SoC	2	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	10 (43.5)	16.14	(4.14, NE)	2.19	(0.48, 10.02)	0.3127	
	SoC	16	2 (12.5)	NE	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	12 (57.1)	4.57	(4.14, 8.14)	NE	(NE, NE)	NE	
	SoC	8	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.

95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).

A Cox proportional model was applied with factor treatment.

Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.

No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.

Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	5 (35.7)	16.14	(4.14, NE)	0.72	(0.11, 4.50)	0.7230	
	SoC	10	2 (20.0)	12.14	(8.14, 12.14)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QSWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by age ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								0.8799

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QSWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by age ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	17 (58.6)	4.14	(NE, NE)	2.66	(0.94, 7.55)	0.0656	
	SoC	13	5 (38.5)	12.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QSWIT10_SITC: Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by age ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	2 (33.3)	14.14	(8.14, 20.14)	2.56	(0.23, 29.12)	0.4482	
	SoC	5	1 (20.0)	NE	(3.86, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QSWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by sex ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.3845

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QSWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by sex ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	7 (36.8)	6.14	(4.14, NE)	1.65	(0.42, 6.48)	0.4735	
	SoC	10	3 (30.0)	16.43	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QSWIT10_SITD: Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by sex ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	12 (75.0)	4.14	(4.00, 4.14)	5.48	(0.90, 33.24)	0.0646	
	SoC	8	3 (37.5)	10.14	(3.86, 12.14)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QSWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								0.8102

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QSWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	6 (50.0)	4.14	(4.14, 4.29)	2.13	(0.25, 18.10)	0.4883	
	SoC	2	1 (50.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QSWIT10_SITB: Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by region
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	13 (56.5)	4.14	(4.00, 8.14)	2.72	(0.92, 8.02)	0.0698	
	SoC	16	5 (31.3)	12.14	(3.86, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QSWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								0.7125

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QSWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	11 (52.4)	4.14	(4.14, 20.14)	2.07	(0.57, 7.49)	0.2669	
	SoC	8	3 (37.5)	16.43	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QSWIT10_SITA: Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by number of transfusions previous year
 ITT

Time to increase of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	8 (57.1)	4.14	(3.86, 4.29)	5.40	(1.06, 27.57)	0.0425	*
	SoC	10	3 (30.0)	12.14	(3.86, 12.14)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QGWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								0.3506

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QGWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by age ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	6 (20.7)	NE	(NE, NE)	0.39	(0.11, 1.40)	0.1470	
	SoC	13	4 (30.8)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QGWD10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by age ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	1 (16.7)	NE	(4.00, NE)	0.23	(0.02, 2.76)	0.2435	
	SoC	5	2 (40.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QGWD10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL
 (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QGWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	2 (16.7)	NE	(16.14, NE)	NE	(NE, NE)	NE	
	SoC	2	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QGWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Global Health Status/QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	5 (21.7)	NE	(NE, NE)	0.31	(0.09, 1.06)	0.0630	
	SoC	16	6 (37.5)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QYWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QYWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	1 (8.3)	NE	(NE, NE)	NE	(NE, NE)	NE	
	SoC	2	1 (50.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QYWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Physical Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	5 (21.7)	NE	(NE, NE)	0.44	(0.12, 1.61)	0.2132	
	SoC	16	5 (31.3)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								0.6875

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by age ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	5 (17.2)	NE	(NE, NE)	0.16	(0.05, 0.51)	0.0019	*
	SoC	13	8 (61.5)	4.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by age ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	2 (33.3)	NE	(4.29, NE)	0.19	(0.02, 2.15)	0.1814	
	SoC	5	3 (60.0)	4.14	(3.86, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by sex
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.8743

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by sex
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	4 (21.1)	NE	(NE, NE)	0.16	(0.04, 0.59)	0.0062	*
	SoC	10	6 (60.0)	4.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by sex
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	3 (18.8)	NE	(20.29, NE)	0.23	(0.04, 1.26)	0.0905	
	SoC	8	5 (62.5)	4.14	(3.86, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	1 (8.3)	NE	(20.29, NE)	NE	(NE, NE)	NE	
	SoC	2	2 (100.0)	4.14	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	6 (26.1)	NE	(NE, NE)	0.27	(0.09, 0.79)	0.0172	*
	SoC	16	9 (56.3)	4.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWDT10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								0.2066

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWDT10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	2 (9.5)	NE	(NE, NE)	0.09	(0.02, 0.48)	0.0052	*
	SoC	8	4 (50.0)	4.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QRWDT10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Role Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	5 (35.7)	NE	(8.14, NE)	0.25	(0.07, 0.83)	0.0234	*
	SoC	10	7 (70.0)	4.14	(3.86, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	9 (31.0)	NE	(20.43, NE)	0.42	(0.14, 1.20)	0.1035	
	SoC	13	6 (46.2)	16.43	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWDT10_SITC: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by age
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	2 (33.3)	NE	(8.43, NE)	NE	(NE, NE)	NE	
	SoC	5	4 (80.0)	4.14	(4.14, 8.29)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by sex
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.4714

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by sex
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	8 (42.1)	NE	(12.14, NE)	0.29	(0.09, 0.93)	0.0379	*
	SoC	10	6 (60.0)	8.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by sex
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	3 (18.8)	NE	(4.14, NE)	0.23	(0.03, 2.07)	0.1890	
	SoC	8	4 (50.0)	8.29	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	1 (8.3)	NE	(20.43, NE)	NE	(NE, NE)	NE	
	SoC	2	1 (50.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	10 (43.5)	NE	(12.00, NE)	0.42	(0.16, 1.06)	0.0656	
	SoC	16	9 (56.3)	8.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWDT10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by number of transfusions previous year
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								0.0876

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWDT10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by number of transfusions previous year
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	8 (38.1)	NE	(12.00, NE)	0.54	(0.14, 2.05)	0.3628	
	SoC	8	3 (37.5)	16.43	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.

95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).

A Cox proportional model was applied with factor treatment.

Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.

No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.

Source Data: apro_tte, created on: 22SEP2023

Table PT1QTWDT10_SITA: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)
 by number of transfusions previous year
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Cognitive Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	3 (21.4)	NE	(26.14, NE)	0.14	(0.03, 0.69)	0.0156	*
	SoC	10	7 (70.0)	6.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QSWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by sex ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QSWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by sex ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	8 (42.1)	NE	(8.14, NE)	0.87	(0.22, 3.45)	0.8378	
	SoC	10	3 (30.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QSWDT10_SITD: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by sex ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	0 (0.0)	NE	(NE, NE)	NE	(NE, NE)	NE	
	SoC	8	3 (37.5)	8.29	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QSWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QSWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	1 (8.3)	NE	(NE, NE)	NE	(NE, NE)	NE	
	SoC	2	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1QSWDT10_SITB: Time to decrease of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored) by region
 ITT

Time to decrease of at least 10 points in change from baseline of QLQ-C30 Social Functioning (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	7 (30.4)	NE	(20.43, NE)	0.56	(0.18, 1.72)	0.3139	
	SoC	16	6 (37.5)	8.29	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LTWC_SICC: Change from baseline in LASA Total Score (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.3256

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SICC: Change from baseline in LASA Total Score (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 60	Peg	Week 4	29	26 (89.7)	36.08 (10.09)	(15.55, 56.62)	44.52 (18.80)	(6.28, 82.77)	0.0239	*
		Week 8	29	24 (82.8)	42.96 (10.57)	(21.41, 64.51)	57.93 (19.14)	(18.88, 96.97)	0.0050	*
		Week 12	29	26 (89.7)	51.31 (10.20)	(30.56, 72.05)	68.93 (18.95)	(30.37, 107.48)	0.0009	*
		Week 16	29	25 (86.2)	46.77 (10.79)	(24.82, 68.72)	45.80 (19.13)	(6.87, 84.73)	0.0225	*
		Week 20	29	27 (93.1)	53.49 (9.66)	(33.86, 73.12)	63.14 (18.19)	(26.17, 100.11)	0.0014	*
	Week 26	29	23 (79.3)	57.04 (11.08)	(34.38, 79.70)	65.77 (20.65)	(23.54, 107.99)	0.0034	*	
	SoC	Week 4	13	11 (84.6)	-8.44 (15.61)	(-40.19, 23.31)				
		Week 8	13	11 (84.6)	-14.97 (15.68)	(-46.95, 17.01)				
		Week 12	13	11 (84.6)	-17.62 (15.75)	(-49.66, 14.41)				
		Week 16	13	12 (92.3)	0.97 (15.62)	(-30.80, 32.74)				
		Week 20	13	11 (84.6)	-9.65 (15.25)	(-40.65, 21.35)				
	Week 26	13	10 (76.9)	-8.73 (17.13)	(-43.76, 26.30)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SICC: Change from baseline in LASA Total Score (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	27.56 (19.35)	(-22.18, 77.29)	60.32 (32.00)	(-21.94, 142.58)	0.1181	
		Week 8	6	5 (83.3)	54.65 (11.41)	(25.31, 83.99)	69.31 (18.88)	(20.78, 117.83)	0.0144	*
		Week 12	6	5 (83.3)	-10.92 (34.41)	(-99.37, 77.52)	47.01 (56.91)	(-99.28, 193.29)	0.4464	
		Week 16	6	5 (83.3)	24.85 (34.47)	(-63.74, 113.45)	78.67 (55.52)	(-64.04, 221.38)	0.2157	
		Week 20	6	5 (83.3)	51.45 (12.53)	(19.24, 83.66)	67.77 (20.19)	(15.88, 119.66)	0.0202	*
	Week 26	6	4 (66.7)	70.36 (22.59)	(-26.85, 167.58)	111.76 (43.76)	(-76.54, 300.06)	0.1252		
	SoC	Week 4	5	4 (80.0)	-32.76 (21.39)	(-87.74, 22.23)				
		Week 8	5	4 (80.0)	-14.65 (12.62)	(-47.09, 17.78)				
		Week 12	5	4 (80.0)	-57.93 (38.04)	(-155.71, 39.85)				
		Week 16	5	4 (80.0)	-53.82 (39.48)	(-155.30, 47.67)				
Week 20		5	4 (80.0)	-16.32 (14.35)	(-53.22, 20.58)					
Week 26	5	2 (40.0)	-41.40 (33.94)	(-187.41, 104.62)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SIHC: Change from baseline in LASA Total Score (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Total Score (censored)	< 60	Baseline	Peg	29	29 (100.0)	182.24 (62.65)	51.0	190.00	280.0	
			SoC	13	12 (92.3)	204.25 (48.54)	135.0	215.00	280.0	
		Week 4	Peg	29	26 (89.7)	218.65 (59.08)	32.0	220.00	300.0	
			SoC	13	12 (92.3)	194.08 (64.42)	79.0	204.00	292.0	
		Week 8	Peg	29	24 (82.8)	226.50 (57.13)	70.0	240.00	300.0	
			SoC	13	11 (84.6)	183.18 (70.76)	60.0	200.00	270.0	
		Week 12	Peg	29	26 (89.7)	235.69 (62.56)	40.0	240.00	300.0	
			SoC	13	11 (84.6)	181.73 (61.22)	104.0	150.00	280.0	
		Week 16	Peg	29	25 (86.2)	234.28 (61.20)	70.0	260.00	300.0	
			SoC	13	12 (92.3)	202.83 (70.05)	100.0	204.00	300.0	
		Week 20	Peg	29	27 (93.1)	239.33 (56.98)	30.0	244.00	300.0	
			SoC	13	12 (92.3)	193.58 (63.53)	90.0	204.00	289.0	
		Week 26	Peg	29	23 (79.3)	237.78 (64.15)	40.0	260.00	300.0	
			SoC	13	10 (76.9)	189.60 (64.04)	95.0	190.00	282.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SIHC: Change from baseline in LASA Total Score (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Total Score (censored)	>= 60	Baseline	Peg	6	6 (100.0)	207.00 (34.31)	167.0	209.50	255.0	
			SoC	5	4 (80.0)	162.50 (45.73)	110.0	160.00	220.0	
		Week 4	Peg	6	5 (83.3)	226.40 (48.42)	160.0	216.00	291.0	
			SoC	5	5 (100.0)	158.00 (47.64)	90.0	150.00	220.0	
		Week 8	Peg	6	5 (83.3)	256.00 (40.01)	195.0	261.00	300.0	
			SoC	5	5 (100.0)	170.80 (44.20)	120.0	150.00	224.0	
		Week 12	Peg	6	5 (83.3)	166.20 (76.58)	30.0	200.00	208.0	
			SoC	5	5 (100.0)	164.80 (39.31)	120.0	150.00	224.0	
		Week 16	Peg	6	5 (83.3)	192.60 (97.36)	30.0	208.00	270.0	
			SoC	5	5 (100.0)	160.80 (38.38)	120.0	150.00	224.0	
		Week 20	Peg	6	5 (83.3)	243.60 (53.84)	166.0	270.00	300.0	
			SoC	5	5 (100.0)	164.80 (39.31)	120.0	150.00	224.0	
		Week 26	Peg	6	4 (66.7)	259.25 (37.70)	209.0	264.00	300.0	
			SoC	5	3 (60.0)	164.67 (53.53)	120.0	150.00	224.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SIHC: Change from baseline in LASA Total Score (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in LASA Total Score (censored)	Week 4	Peg	29	26 (89.7)	35.81 (59.76)	-55.0	20.00	180.0	0.77 (0.38) [0.07, 1.58]
		SoC	13	11 (84.6)	-9.27 (49.71)	-141.0	6.00	40.0	
	Week 8	Peg	29	24 (82.8)	42.92 (57.30)	-30.0	20.00	159.0	1.04 (0.40) [0.32, 1.89]
		SoC	13	11 (84.6)	-16.00 (51.25)	-114.0	0.00	60.0	
	Week 12	Peg	29	26 (89.7)	50.54 (60.86)	-55.0	38.50	189.0	1.13 (0.40) [0.43, 1.99]
		SoC	13	11 (84.6)	-17.45 (52.59)	-116.0	0.00	60.0	
	Week 16	Peg	29	25 (86.2)	47.24 (64.29)	-45.0	22.00	189.0	0.80 (0.38) [0.11, 1.59]
		SoC	13	12 (92.3)	-1.42 (48.12)	-120.0	5.50	60.0	
	Week 20	Peg	29	27 (93.1)	53.56 (61.87)	-30.0	30.00	189.0	1.05 (0.39) [0.35, 1.89]
		SoC	13	11 (84.6)	-9.82 (50.01)	-130.0	0.00	60.0	
	Week 26	Peg	29	23 (79.3)	56.70 (63.57)	-65.0	56.00	189.0	1.02 (0.42) [0.28, 1.91]
		SoC	13	10 (76.9)	-6.00 (50.45)	-125.0	1.00	60.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SIHC: Change from baseline in LASA Total Score (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline >= 60 in LASA Total Score (censored)	Week 4	Peg	6	5 (83.3)	12.00 (26.58)	-16.0	3.00	44.0	0.72 (0.84) [-0.66, 2.66]	
		SoC	5	4 (80.0)	-20.00 (51.64)	-80.0	-20.00	40.0		
	Week 8	Peg	6	5 (83.3)	41.60 (19.32)	28.0	31.00	74.0	1.47 (0.96) [0.15, 3.89]	
		SoC	5	4 (80.0)	-5.00 (36.97)	-50.0	-5.00	40.0		
	Week 12	Peg	6	5 (83.3)	-48.20 (103.11)	-225.0	-6.00	39.0	-0.38 (0.81) [-2.14, 1.07]	
		SoC	5	4 (80.0)	-12.50 (41.13)	-50.0	-20.00	40.0		
	Week 16	Peg	6	5 (83.3)	-9.60 (126.33)	-225.0	23.00	95.0	0.07 (0.79) [-1.49, 1.68]	
		SoC	5	4 (80.0)	-17.50 (46.46)	-60.0	-25.00	40.0		
	Week 20	Peg	6	5 (83.3)	41.40 (43.45)	-1.0	45.00	100.0	1.13 (0.89) [-0.20, 3.32]	
		SoC	5	4 (80.0)	-12.50 (41.13)	-50.0	-20.00	40.0		
	Week 26	Peg	6	4 (66.7)	49.00 (43.03)	-4.0	49.50	101.0	1.44 (1.55) [-0.29, 5.46]	
		SoC	5	2 (40.0)	-25.00 (35.36)	-50.0	-25.00	0.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SICD: Change from baseline in LASA Total Score (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.1583

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SICD: Change from baseline in LASA Total Score (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Male	Peg	Week 4	19	16 (84.2)	35.49 (13.88)	(6.54, 64.43)	73.19 (24.05)	(23.03, 123.35)	0.0064	*
		Week 8	19	15 (78.9)	37.46 (12.96)	(10.33, 64.60)	75.84 (22.21)	(29.35, 122.32)	0.0029	*
		Week 12	19	17 (89.5)	25.33 (15.41)	(-6.72, 57.39)	72.79 (27.50)	(15.61, 129.97)	0.0151	*
		Week 16	19	16 (84.2)	26.82 (18.10)	(-10.82, 64.45)	53.25 (30.60)	(-10.38, 116.87)	0.0964	
		Week 20	19	17 (89.5)	48.91 (11.29)	(25.44, 72.39)	90.28 (20.06)	(48.55, 132.00)	0.0002	*
	Week 26	19	14 (73.7)	56.18 (12.68)	(29.31, 83.06)	99.28 (23.55)	(49.35, 149.21)	0.0007	*	
	SoC	Week 4	10	8 (80.0)	-37.70 (19.78)	(-78.96, 3.55)				
		Week 8	10	8 (80.0)	-38.38 (18.01)	(-76.07, -0.68)				
		Week 12	10	8 (80.0)	-47.46 (22.74)	(-94.74, -0.18)				
		Week 16	10	9 (90.0)	-26.43 (24.64)	(-77.67, 24.81)				
		Week 20	10	8 (80.0)	-41.36 (16.61)	(-75.92, -6.81)				
Week 26		10	6 (60.0)	-43.10 (19.56)	(-84.56, -1.63)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SICD: Change from baseline in LASA Total Score (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Female	Peg	Week 4	16	15 (93.8)	31.31 (10.86)	(8.50, 54.12)	25.25 (23.00)	(-23.08, 73.59)	0.2868	
		Week 8	16	14 (87.5)	50.88 (11.97)	(25.63, 76.13)	47.25 (25.28)	(-6.08, 100.58)	0.0789	
		Week 12	16	14 (87.5)	52.91 (13.46)	(24.52, 81.30)	43.25 (28.42)	(-16.72, 103.22)	0.1465	
		Week 16	16	14 (87.5)	57.30 (13.16)	(29.52, 85.07)	59.34 (27.80)	(0.68, 118.00)	0.0477	*
		Week 20	16	15 (93.8)	59.31 (12.47)	(33.11, 85.51)	57.28 (26.43)	(1.75, 112.80)	0.0439	*
	Week 26	16	13 (81.3)	60.82 (15.39)	(28.00, 93.63)	58.79 (33.28)	(-12.15, 129.74)	0.0977		
	SoC	Week 4	8	7 (87.5)	6.05 (19.02)	(-33.90, 46.01)				
		Week 8	8	7 (87.5)	3.63 (20.23)	(-39.04, 46.30)				
		Week 12	8	7 (87.5)	9.66 (22.74)	(-38.32, 57.65)				
		Week 16	8	7 (87.5)	-2.04 (22.25)	(-48.98, 44.90)				
		Week 20	8	7 (87.5)	2.04 (21.85)	(-43.86, 47.93)				
Week 26		8	6 (75.0)	2.02 (27.13)	(-55.81, 59.86)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SIHD: Change from baseline in LASA Total Score (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Total Score (censored)	Male	Baseline	Peg	19	19 (100.0)	195.63 (52.66)	110.0	210.00	280.0	
			SoC	10	9 (90.0)	190.11 (45.07)	135.0	180.00	260.0	
		Week 4	Peg	19	16 (84.2)	224.81 (55.74)	110.0	218.00	300.0	
			SoC	10	9 (90.0)	155.44 (58.07)	79.0	150.00	255.0	
		Week 8	Peg	19	15 (78.9)	231.40 (56.03)	110.0	244.00	300.0	
			SoC	10	9 (90.0)	155.67 (63.55)	60.0	141.00	260.0	
		Week 12	Peg	19	17 (89.5)	216.41 (75.48)	30.0	210.00	300.0	
			SoC	10	9 (90.0)	147.22 (36.23)	104.0	141.00	224.0	
		Week 16	Peg	19	16 (84.2)	221.56 (75.40)	30.0	240.00	300.0	
			SoC	10	10 (100.0)	170.50 (63.56)	100.0	150.00	300.0	
		Week 20	Peg	19	17 (89.5)	239.12 (50.85)	150.0	240.00	300.0	
			SoC	10	9 (90.0)	154.44 (45.56)	90.0	145.00	224.0	
		Week 26	Peg	19	14 (73.7)	242.14 (54.38)	140.0	256.00	300.0	
			SoC	10	7 (70.0)	147.14 (43.25)	95.0	141.00	224.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SIHD: Change from baseline in LASA Total Score (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Total Score (censored)	Female	Baseline	Peg	16	16 (100.0)	175.63 (66.05)	51.0	190.00	270.0	
			SoC	8	7 (87.5)	198.57 (59.00)	110.0	210.00	280.0	
	Week 4	Peg	16	15 (93.8)	214.67 (59.46)	32.0	220.00	280.0		
		SoC	8	8 (100.0)	215.00 (49.75)	150.0	214.00	292.0		
	Week 8	Peg	16	14 (87.5)	231.79 (56.24)	70.0	240.00	300.0		
		SoC	8	7 (87.5)	209.71 (49.33)	150.0	208.00	270.0		
	Week 12	Peg	16	14 (87.5)	234.29 (60.73)	40.0	240.00	300.0		
		SoC	8	7 (87.5)	214.00 (52.59)	150.0	208.00	280.0		
	Week 16	Peg	16	14 (87.5)	233.93 (61.16)	70.0	265.00	287.0		
		SoC	8	7 (87.5)	219.00 (57.82)	150.0	208.00	295.0		
	Week 20	Peg	16	15 (93.8)	241.00 (62.49)	30.0	269.00	285.0		
		SoC	8	8 (100.0)	219.63 (51.97)	150.0	224.00	289.0		
	Week 26	Peg	16	13 (81.3)	239.69 (69.46)	40.0	260.00	300.0		
		SoC	8	6 (75.0)	226.67 (49.84)	150.0	229.00	282.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SIHD: Change from baseline in LASA Total Score (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in LASA Total Score (censored)	Male	Week 4	Peg	19	16 (84.2)	33.13 (63.99)	-49.0	20.00	180.0	1.07 (0.49) [0.22, 2.14]
			SoC	10	8 (80.0)	-34.00 (53.32)	-141.0	-18.50	20.0	
		Week 8	Peg	19	15 (78.9)	36.13 (51.68)	-30.0	28.00	150.0	1.35 (0.51) [0.48, 2.50]
			SoC	10	8 (80.0)	-34.25 (47.71)	-114.0	-30.00	25.0	
		Week 12	Peg	19	17 (89.5)	20.12 (87.49)	-225.0	27.00	180.0	0.80 (0.47) [-0.04, 1.81]
			SoC	10	8 (80.0)	-43.75 (43.31)	-116.0	-45.00	6.0	
		Week 16	Peg	19	16 (84.2)	25.44 (88.63)	-225.0	21.00	160.0	0.64 (0.45) [-0.18, 1.59]
			SoC	10	9 (90.0)	-25.56 (46.94)	-120.0	-21.00	40.0	
		Week 20	Peg	19	17 (89.5)	46.88 (60.19)	-19.0	30.00	180.0	1.44 (0.50) [0.59, 2.57]
			SoC	10	8 (80.0)	-35.63 (42.72)	-130.0	-28.00	6.0	
		Week 26	Peg	19	14 (73.7)	51.29 (58.53)	-19.0	37.50	160.0	1.41 (0.58) [0.45, 2.73]
			SoC	10	6 (60.0)	-31.67 (50.17)	-125.0	-10.50	6.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SIHD: Change from baseline in LASA Total Score (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in LASA Total Score (censored)	Female	Week 4	Peg	16	15 (93.8)	30.73 (48.56)	-55.0	18.00	139.0	0.39 (0.49) [-0.52, 1.40]
			SoC	8	7 (87.5)	12.86 (28.73)	-42.0	12.00	40.0	
		Week 8	Peg	16	14 (87.5)	49.71 (54.27)	-20.0	38.00	159.0	0.76 (0.51) [-0.15, 1.85]
			SoC	8	7 (87.5)	11.14 (34.39)	-42.0	0.00	60.0	
		Week 12	Peg	16	14 (87.5)	52.21 (59.24)	-20.0	50.00	189.0	0.67 (0.50) [-0.24, 1.75]
			SoC	8	7 (87.5)	15.43 (33.16)	-42.0	20.00	60.0	
		Week 16	Peg	16	14 (87.5)	51.86 (64.11)	-37.0	43.50	189.0	0.54 (0.50) [-0.38, 1.59]
			SoC	8	7 (87.5)	20.43 (33.59)	-42.0	30.00	60.0	
		Week 20	Peg	16	15 (93.8)	57.07 (59.11)	-30.0	40.00	189.0	0.71 (0.50) [-0.19, 1.78]
			SoC	8	7 (87.5)	18.14 (33.02)	-42.0	30.00	60.0	
		Week 26	Peg	16	13 (81.3)	60.15 (64.28)	-65.0	60.00	189.0	0.78 (0.55) [-0.19, 1.97]
			SoC	8	6 (75.0)	13.33 (34.93)	-42.0	16.00	60.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SICB: Change from baseline in LASA Total Score (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.2170

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SICB: Change from baseline in LASA Total Score (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Latin America	Peg	Week 4	12	11 (91.7)	82.93 (9.48)	(61.48, 104.37)	101.40 (24.52)	(45.93, 156.86)	0.0025	*
		Week 8	12	10 (83.3)	87.52 (9.50)	(65.62, 109.43)	108.63 (23.77)	(53.82, 163.45)	0.0018	*
		Week 12	12	11 (91.7)	67.61 (17.22)	(28.65, 106.57)	106.51 (44.54)	(5.75, 207.28)	0.0405	*
		Week 16	12	10 (83.3)	71.61 (20.57)	(24.18, 119.05)	112.68 (51.48)	(-6.03, 231.38)	0.0600	
		Week 20	12	10 (83.3)	102.75 (10.37)	(78.83, 126.67)	118.50 (25.74)	(59.16, 177.85)	0.0017	*
		Week 26	12	9 (75.0)	104.24 (10.09)	(80.39, 128.09)	112.83 (23.74)	(56.68, 168.97)	0.0021	*
	SoC	Week 4	2	2 (100.0)	-18.47 (22.46)	(-69.28, 32.34)				
		Week 8	2	2 (100.0)	-21.11 (21.61)	(-70.94, 28.72)				
		Week 12	2	2 (100.0)	-38.91 (40.81)	(-131.22, 53.41)				
		Week 16	2	2 (100.0)	-41.06 (46.79)	(-148.97, 66.84)				
		Week 20	2	2 (100.0)	-15.75 (23.43)	(-69.79, 38.28)				
	Week 26	2	2 (100.0)	-8.59 (21.41)	(-59.21, 42.04)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SICB: Change from baseline in LASA Total Score (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Asia Pacific Peg	Peg	Week 4	23	20 (87.0)	9.02 (9.17)	(-9.74, 27.77)	29.08 (15.05)	(-1.70, 59.86)	0.0631	
		Week 8	23	19 (82.6)	23.89 (9.58)	(4.27, 43.52)	42.74 (15.51)	(10.98, 74.50)	0.0102	*
		Week 12	23	20 (87.0)	25.87 (9.74)	(5.95, 45.79)	52.20 (15.99)	(19.50, 84.90)	0.0028	*
		Week 16	23	20 (87.0)	27.74 (10.74)	(5.81, 49.68)	37.95 (17.03)	(3.16, 72.74)	0.0336	*
		Week 20	23	22 (95.7)	32.57 (9.54)	(13.12, 52.02)	50.28 (16.09)	(17.45, 83.10)	0.0039	*
	Week 26	23	18 (78.3)	34.56 (11.70)	(10.42, 58.70)	56.75 (20.76)	(13.90, 99.61)	0.0116	*	
	SoC	Week 4	16	13 (81.3)	-20.06 (11.58)	(-43.74, 3.61)				
		Week 8	16	13 (81.3)	-18.84 (11.70)	(-42.81, 5.12)				
		Week 12	16	13 (81.3)	-26.33 (12.30)	(-51.49, -1.18)				
		Week 16	16	14 (87.5)	-10.20 (12.91)	(-36.56, 16.15)				
Week 20		16	13 (81.3)	-17.71 (12.63)	(-43.47, 8.05)					
Week 26	16	10 (62.5)	-22.19 (16.76)	(-56.77, 12.40)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SIHB: Change from baseline in LASA Total Score (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Total Score (censored)	Latin America	Baseline	Peg	12	12 (100.0)	161.00 (64.75)	51.0	151.00	255.0	
			SoC	2	2 (100.0)	142.50 (10.61)	135.0	142.50	150.0	
		Week 4	Peg	12	11 (91.7)	250.00 (30.22)	210.0	250.00	300.0	
			SoC	2	2 (100.0)	145.50 (6.36)	141.0	145.50	150.0	
		Week 8	Peg	12	10 (83.3)	257.00 (28.69)	210.0	265.00	300.0	
			SoC	2	2 (100.0)	145.50 (6.36)	141.0	145.50	150.0	
		Week 12	Peg	12	11 (91.7)	228.45 (74.05)	30.0	250.00	300.0	
			SoC	2	2 (100.0)	145.50 (6.36)	141.0	145.50	150.0	
		Week 16	Peg	12	10 (83.3)	237.10 (75.66)	30.0	270.00	291.0	
			SoC	2	2 (100.0)	145.50 (6.36)	141.0	145.50	150.0	
		Week 20	Peg	12	10 (83.3)	265.30 (30.93)	210.0	270.00	300.0	
			SoC	2	2 (100.0)	145.50 (6.36)	141.0	145.50	150.0	
		Week 26	Peg	12	9 (75.0)	262.11 (31.31)	194.0	270.00	300.0	
			SoC	2	2 (100.0)	145.50 (6.36)	141.0	145.50	150.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SIHB: Change from baseline in LASA Total Score (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Total Score (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	199.78 (52.59)	60.0	210.00	280.0	
			SoC	16	14 (87.5)	201.14 (49.06)	110.0	215.00	280.0	
		Week 4	Peg	23	20 (87.0)	203.35 (61.70)	32.0	210.00	300.0	
	SoC		16	15 (93.8)	188.53 (63.26)	79.0	200.00	292.0		
		Week 8	Peg	23	19 (82.6)	218.21 (61.37)	70.0	230.00	300.0	
	SoC		16	14 (87.5)	184.14 (65.49)	60.0	204.00	270.0		
		Week 12	Peg	23	20 (87.0)	222.30 (67.46)	40.0	240.00	300.0	
	SoC		16	14 (87.5)	180.86 (57.12)	104.0	165.00	280.0		
		Week 16	Peg	23	20 (87.0)	222.45 (65.74)	70.0	240.00	300.0	
	SoC		16	15 (93.8)	196.47 (66.27)	100.0	200.00	300.0		
		Week 20	Peg	23	22 (95.7)	228.50 (61.01)	30.0	240.00	300.0	
	SoC		16	15 (93.8)	190.40 (59.70)	90.0	200.00	289.0		
		Week 26	Peg	23	18 (78.3)	230.39 (69.66)	40.0	242.00	300.0	
	SoC		16	11 (68.8)	190.82 (63.69)	95.0	200.00	282.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SIHB: Change from baseline in LASA Total Score (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline in LASA Total Score (censored)	Latin America	Week 4	Peg	12	11 (91.7)	79.00 (59.09)	2.0	58.00	180.0	1.25 (0.91)	[-0.26, 3.34]
			SoC	2	2 (100.0)	3.00 (4.24)	0.0	3.00	6.0		
		Week 8	Peg	12	10 (83.3)	82.70 (61.10)	-6.0	81.00	159.0	1.27 (0.93)	[-0.26, 3.43]
			SoC	2	2 (100.0)	3.00 (4.24)	0.0	3.00	6.0		
		Week 12	Peg	12	11 (91.7)	57.45 (115.67)	-225.0	66.00	189.0	0.46 (0.86)	[-1.14, 2.27]
			SoC	2	2 (100.0)	3.00 (4.24)	0.0	3.00	6.0		
		Week 16	Peg	12	10 (83.3)	62.80 (117.80)	-225.0	76.00	189.0	0.49 (0.88)	[-1.13, 2.36]
			SoC	2	2 (100.0)	3.00 (4.24)	0.0	3.00	6.0		
		Week 20	Peg	12	10 (83.3)	99.00 (63.21)	-6.0	90.00	189.0	1.48 (0.96)	[-0.04, 3.74]
			SoC	2	2 (100.0)	3.00 (4.24)	0.0	3.00	6.0		
		Week 26	Peg	12	9 (75.0)	101.33 (53.53)	45.0	88.00	189.0	1.78 (1.03)	[0.25, 4.31]
			SoC	2	2 (100.0)	3.00 (4.24)	0.0	3.00	6.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SIHB: Change from baseline in LASA Total Score (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in LASA Total Score (censored)	Asia	Week 4	Peg	23	20 (87.0)	6.10 (33.91)	-55.0	10.00	90.0	0.48 (0.37) [-0.22, 1.25]
			SoC	16	13 (81.3)	-14.46 (52.04)	-141.0	0.00	40.0	
	Pacific	Week 8	Peg	23	19 (82.6)	21.63 (32.47)	-30.0	20.00	90.0	0.90 (0.39) [0.19, 1.74]
			SoC	16	13 (81.3)	-15.54 (50.09)	-114.0	-10.00	60.0	
	Week 12	Peg	23	20 (87.0)	22.05 (41.89)	-55.0	23.50	90.0	0.87 (0.39) [0.18, 1.70]	
		SoC	16	13 (81.3)	-19.08 (51.59)	-116.0	-5.00	60.0		
	Week 16	Peg	23	20 (87.0)	25.25 (47.13)	-45.0	15.00	120.0	0.64 (0.37) [-0.04, 1.42]	
		SoC	16	14 (87.5)	-6.64 (50.03)	-120.0	2.50	60.0		
	Week 20	Peg	23	22 (95.7)	30.14 (43.18)	-30.0	21.00	120.0	0.91 (0.38) [0.23, 1.73]	
		SoC	16	13 (81.3)	-12.62 (49.72)	-130.0	-15.00	60.0		
	Week 26	Peg	23	18 (78.3)	32.67 (50.55)	-65.0	25.00	110.0	0.84 (0.43) [0.07, 1.76]	
		SoC	16	10 (62.5)	-11.60 (52.06)	-125.0	0.00	60.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SICA: Change from baseline in LASA Total Score (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.5329

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SICA: Change from baseline in LASA Total Score (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 4	21	18 (85.7)	24.15 (13.37)	(-3.74, 52.05)	52.71 (28.78)	(-7.32, 112.74)	0.0820	
		Week 8	21	17 (81.0)	37.59 (12.34)	(11.76, 63.42)	63.79 (25.95)	(9.49, 118.10)	0.0237	*
		Week 12	21	18 (85.7)	21.65 (17.19)	(-14.20, 57.50)	73.79 (36.99)	(-3.38, 150.95)	0.0599	
		Week 16	21	17 (81.0)	20.85 (18.70)	(-18.15, 59.85)	35.09 (37.09)	(-42.27, 112.45)	0.3553	
		Week 20	21	18 (85.7)	48.34 (12.27)	(22.76, 73.93)	84.57 (26.47)	(29.36, 139.77)	0.0045	*
	Week 26	21	15 (71.4)	48.90 (16.00)	(14.81, 83.00)	88.42 (39.19)	(4.90, 171.95)	0.0394	*	
	SoC	Week 4	8	5 (62.5)	-28.55 (25.43)	(-81.61, 24.50)				
		Week 8	8	5 (62.5)	-26.20 (22.79)	(-73.91, 21.50)				
		Week 12	8	5 (62.5)	-52.14 (32.69)	(-120.34, 16.06)				
		Week 16	8	6 (75.0)	-14.24 (31.74)	(-80.45, 51.96)				
		Week 20	8	5 (62.5)	-36.23 (23.37)	(-84.98, 12.53)				
Week 26		8	3 (37.5)	-39.52 (35.77)	(-115.77, 36.73)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SICA: Change from baseline in LASA Total Score (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	42.08 (12.03)	(16.98, 67.17)	45.08 (18.27)	(6.97, 83.19)	0.0228	*
		Week 8	14	12 (85.7)	49.88 (13.26)	(22.12, 77.64)	56.34 (19.69)	(15.14, 97.55)	0.0100	*
		Week 12	14	13 (92.9)	52.31 (11.79)	(27.71, 76.91)	50.11 (17.95)	(12.68, 87.55)	0.0112	*
		Week 16	14	13 (92.9)	56.01 (12.32)	(30.32, 81.71)	51.13 (18.74)	(12.03, 90.23)	0.0130	*
		Week 20	14	14 (100.0)	55.76 (12.75)	(29.25, 82.26)	53.21 (19.83)	(11.98, 94.45)	0.0139	*
	Week 26	14	12 (85.7)	64.02 (12.19)	(38.42, 89.62)	63.27 (18.62)	(24.16, 102.38)	0.0032	*	
	SoC	Week 4	10	10 (100.0)	-3.00 (13.72)	(-31.62, 25.62)				
		Week 8	10	10 (100.0)	-6.46 (14.53)	(-36.87, 23.95)				
		Week 12	10	10 (100.0)	2.20 (13.46)	(-25.87, 30.27)				
		Week 16	10	10 (100.0)	4.88 (14.06)	(-24.44, 34.20)				
		Week 20	10	10 (100.0)	2.54 (15.10)	(-28.86, 33.94)				
		Week 26	10	9 (90.0)	0.75 (14.07)	(-28.81, 30.31)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SIHA: Change from baseline in LASA Total Score (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Total Score (censored)	< 4	Baseline	Peg	21	21 (100.0)	179.29 (64.39)	51.0	200.00	270.0	
			SoC	8	6 (75.0)	208.33 (44.35)	135.0	220.00	260.0	
		Week 4	Peg	21	18 (85.7)	210.61 (64.14)	32.0	217.50	291.0	
			SoC	8	7 (87.5)	183.57 (61.32)	79.0	180.00	255.0	
		Week 8	Peg	21	17 (81.0)	226.29 (64.12)	70.0	240.00	300.0	
			SoC	8	6 (75.0)	178.50 (61.19)	106.0	175.50	260.0	
		Week 12	Peg	21	18 (85.7)	208.78 (80.36)	30.0	240.00	300.0	
			SoC	8	6 (75.0)	153.17 (43.39)	104.0	145.50	224.0	
		Week 16	Peg	21	17 (81.0)	209.59 (77.94)	30.0	240.00	275.0	
			SoC	8	7 (87.5)	187.86 (69.19)	100.0	160.00	300.0	
		Week 20	Peg	21	18 (85.7)	231.56 (60.11)	30.0	240.00	300.0	
			SoC	8	7 (87.5)	177.14 (54.66)	90.0	180.00	240.0	
		Week 26	Peg	21	15 (71.4)	226.93 (69.68)	40.0	244.00	300.0	
			SoC	8	4 (50.0)	160.00 (55.02)	95.0	160.50	224.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SIHA: Change from baseline in LASA Total Score (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Total Score (censored)	>= 4	Baseline	Peg	14	14 (100.0)	197.29 (50.54)	120.0	190.00	280.0	
			SoC	10	10 (100.0)	185.10 (53.29)	110.0	170.00	280.0	
	Week 4	Peg	14	13 (92.9)	232.77 (44.01)	160.0	240.00	300.0		
		SoC	10	10 (100.0)	183.40 (63.92)	90.0	175.00	292.0		
	Week 8	Peg	14	12 (85.7)	239.08 (40.60)	150.0	240.00	300.0		
		SoC	10	10 (100.0)	179.80 (66.40)	60.0	175.00	270.0		
	Week 12	Peg	14	13 (92.9)	246.23 (42.02)	171.0	240.00	300.0		
		SoC	10	10 (100.0)	190.40 (57.77)	120.0	175.00	280.0		
	Week 16	Peg	14	13 (92.9)	250.54 (46.11)	151.0	270.00	300.0		
		SoC	10	10 (100.0)	192.30 (64.37)	120.0	175.00	295.0		
	Week 20	Peg	14	14 (100.0)	250.86 (49.40)	150.0	269.50	300.0		
		SoC	10	10 (100.0)	190.70 (62.20)	120.0	175.00	289.0		
	Week 26	Peg	14	12 (85.7)	258.50 (44.48)	150.0	269.00	300.0		
		SoC	10	9 (90.0)	194.44 (62.96)	120.0	200.00	282.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SIHA: Change from baseline in LASA Total Score (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in LASA Total Score (censored)	Week 4	Peg	21	18 (85.7)	25.39 (60.29)	-55.0	14.00	160.0	0.92 (0.55) [-0.07, 2.12]
		SoC	8	5 (62.5)	-33.00 (64.37)	-141.0	-10.00	20.0	
	Week 8	Peg	21	17 (81.0)	38.29 (52.21)	-30.0	20.00	159.0	1.22 (0.58) [0.22, 2.50]
		SoC	8	5 (62.5)	-28.60 (55.15)	-114.0	-10.00	25.0	
	Week 12	Peg	21	18 (85.7)	23.56 (87.87)	-225.0	33.50	189.0	0.98 (0.56) [-0.02, 2.18]
		SoC	8	5 (62.5)	-59.00 (46.13)	-116.0	-60.00	6.0	
	Week 16	Peg	21	17 (81.0)	25.18 (91.96)	-225.0	10.00	189.0	0.59 (0.51) [-0.35, 1.66]
		SoC	8	6 (75.0)	-26.50 (57.18)	-120.0	-12.50	40.0	
	Week 20	Peg	21	18 (85.7)	50.17 (59.78)	-30.0	35.00	189.0	1.53 (0.59) [0.54, 2.87]
		SoC	8	5 (62.5)	-42.80 (52.03)	-130.0	-35.00	6.0	
	Week 26	Peg	21	15 (71.4)	48.93 (67.90)	-65.0	45.00	189.0	1.23 (0.72) [-0.01, 2.83]
		SoC	8	3 (37.5)	-39.67 (73.96)	-125.0	0.00	6.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWC_SIHA: Change from baseline in LASA Total Score (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in LASA Total Score (censored)	Week 4	Peg	14	13 (92.9)	41.08 (50.69)	-7.0	30.00	180.0	0.90 (0.47) [0.08, 1.92]
		SoC	10	10 (100.0)	-1.70 (38.31)	-80.0	5.00	40.0	
	Week 8	Peg	14	12 (85.7)	48.92 (54.44)	-20.0	38.00	150.0	1.05 (0.49) [0.21, 2.13]
		SoC	10	10 (100.0)	-5.30 (43.13)	-81.0	0.00	60.0	
	Week 12	Peg	14	13 (92.9)	49.92 (57.38)	-20.0	39.00	180.0	0.88 (0.47) [0.06, 1.90]
		SoC	10	10 (100.0)	5.30 (34.17)	-50.0	0.00	60.0	
	Week 16	Peg	14	13 (92.9)	54.23 (53.98)	-18.0	30.00	150.0	0.96 (0.47) [0.13, 1.99]
		SoC	10	10 (100.0)	7.20 (36.68)	-50.0	7.50	60.0	
	Week 20	Peg	14	14 (100.0)	53.57 (60.05)	-19.0	30.00	180.0	0.90 (0.46) [0.09, 1.90]
		SoC	10	10 (100.0)	5.60 (35.70)	-50.0	4.50	60.0	
	Week 26	Peg	14	12 (85.7)	63.83 (51.01)	-19.0	74.00	150.0	1.34 (0.52) [0.47, 2.53]
		SoC	10	9 (90.0)	1.00 (35.53)	-50.0	0.00	60.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SICC: Change from baseline in LASA Activity Level (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.3831

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SICC: Change from baseline in LASA Activity Level (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 60	Peg	Week 4	29	26 (89.7)	13.33 (3.64)	(5.92, 20.73)	16.58 (6.82)	(2.71, 30.45)	0.0206	*
		Week 8	29	24 (82.8)	15.42 (3.86)	(7.54, 23.30)	20.17 (7.04)	(5.81, 34.54)	0.0074	*
		Week 12	29	26 (89.7)	19.00 (3.45)	(11.99, 26.01)	24.67 (6.43)	(11.59, 37.75)	0.0005	*
		Week 16	29	25 (86.2)	16.52 (3.83)	(8.73, 24.32)	16.35 (6.84)	(2.44, 30.27)	0.0227	*
		Week 20	29	27 (93.1)	19.91 (3.30)	(13.20, 26.61)	22.40 (6.24)	(9.72, 35.09)	0.0010	*
		Week 26	29	23 (79.3)	21.39 (3.79)	(13.65, 29.14)	25.17 (7.11)	(10.64, 39.71)	0.0014	*
	SoC	Week 4	13	11 (84.6)	-3.26 (5.65)	(-14.75, 8.24)				
		Week 8	13	11 (84.6)	-4.75 (5.75)	(-16.48, 6.99)				
		Week 12	13	11 (84.6)	-5.67 (5.33)	(-16.53, 5.18)				
		Week 16	13	12 (92.3)	0.17 (5.57)	(-11.15, 11.49)				
		Week 20	13	11 (84.6)	-2.50 (5.22)	(-13.12, 8.12)				
		Week 26	13	10 (76.9)	-3.78 (5.88)	(-15.80, 8.24)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SICC: Change from baseline in LASA Activity Level (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	9.46 (6.98)	(-8.49, 27.40)	18.10 (11.15)	(-10.58, 46.77)	0.1657	
		Week 8	6	5 (83.3)	19.77 (3.75)	(10.12, 29.41)	22.12 (5.99)	(6.71, 37.52)	0.0141	*
		Week 12	6	5 (83.3)	-4.27 (10.00)	(-29.97, 21.43)	9.71 (15.97)	(-31.33, 50.76)	0.5696	
		Week 16	6	5 (83.3)	6.88 (10.56)	(-20.26, 34.01)	22.90 (16.84)	(-20.39, 66.20)	0.2320	
		Week 20	6	5 (83.3)	19.42 (3.39)	(10.72, 28.13)	23.46 (5.40)	(9.57, 37.35)	0.0074	*
	Week 26	6	4 (66.7)	25.08 (7.35)	(-6.54, 56.71)	30.50 (15.44)	(-35.91, 96.91)	0.1868		
	SoC	Week 4	5	4 (80.0)	-8.64 (8.15)	(-29.58, 12.30)				
		Week 8	5	4 (80.0)	-2.35 (4.38)	(-13.60, 8.90)				
		Week 12	5	4 (80.0)	-13.98 (11.66)	(-43.96, 15.99)				
		Week 16	5	4 (80.0)	-16.03 (12.33)	(-47.72, 15.66)				
		Week 20	5	4 (80.0)	-4.04 (3.96)	(-14.21, 6.13)				
Week 26	5	2 (40.0)	-5.42 (13.09)	(-61.74, 50.91)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SIHC: Change from baseline in LASA Activity Level (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Activity Level (censored)	< 60	Baseline	Peg	29	29 (100.0)	58.48 (20.56)	17.0	60.00	90.0	
			SoC	13	12 (92.3)	67.92 (16.02)	45.0	67.50	95.0	
		Week 4	Peg	29	26 (89.7)	71.88 (20.97)	10.0	77.00	100.0	
			SoC	13	12 (92.3)	63.25 (21.52)	25.0	70.00	100.0	
		Week 8	Peg	29	24 (82.8)	74.13 (19.98)	20.0	80.00	100.0	
			SoC	13	11 (84.6)	60.00 (24.45)	20.0	70.00	90.0	
		Week 12	Peg	29	26 (89.7)	78.08 (21.47)	10.0	80.00	100.0	
			SoC	13	11 (84.6)	59.91 (21.04)	34.0	50.00	90.0	
		Week 16	Peg	29	25 (86.2)	76.64 (21.58)	20.0	80.00	100.0	
			SoC	13	12 (92.3)	66.50 (23.50)	33.0	71.00	100.0	
		Week 20	Peg	29	27 (93.1)	79.59 (18.76)	10.0	80.00	100.0	
			SoC	13	12 (92.3)	63.83 (21.63)	30.0	70.00	96.0	
		Week 26	Peg	29	23 (79.3)	79.26 (21.72)	10.0	83.00	100.0	
			SoC	13	10 (76.9)	62.40 (22.43)	30.0	65.00	94.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SIHC: Change from baseline in LASA Activity Level (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Activity Level (censored)	>= 60	Baseline	Peg	6	6 (100.0)	65.83 (18.00)	35.0	72.50	85.0	
			SoC	5	4 (80.0)	55.00 (10.00)	50.0	50.00	70.0	
		Week 4	Peg	6	5 (83.3)	71.60 (18.53)	50.0	69.00	91.0	
			SoC	5	5 (100.0)	52.00 (14.83)	30.0	50.00	70.0	
		Week 8	Peg	6	5 (83.3)	83.00 (13.34)	64.0	81.00	100.0	
			SoC	5	5 (100.0)	58.00 (16.43)	40.0	50.00	80.0	
		Week 12	Peg	6	5 (83.3)	54.20 (24.96)	10.0	65.00	70.0	
			SoC	5	5 (100.0)	56.00 (15.17)	40.0	50.00	80.0	
		Week 16	Peg	6	5 (83.3)	63.60 (31.90)	10.0	70.00	90.0	
			SoC	5	5 (100.0)	54.00 (15.17)	40.0	50.00	80.0	
		Week 20	Peg	6	5 (83.3)	82.00 (16.43)	60.0	90.00	100.0	
			SoC	5	5 (100.0)	56.00 (15.17)	40.0	50.00	80.0	
		Week 26	Peg	6	4 (66.7)	84.25 (13.57)	68.0	84.50	100.0	
			SoC	5	3 (60.0)	56.67 (20.82)	40.0	50.00	80.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SIHC: Change from baseline in LASA Activity Level (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in LASA Activity Level (censored)	Week 4	Peg	29	26 (89.7)	13.46 (22.69)	-25.0	10.00	70.0	0.83 (0.38) [0.12, 1.64]
		SoC	13	11 (84.6)	-4.18 (15.17)	-45.0	0.00	10.0	
	Week 8	Peg	29	24 (82.8)	15.79 (20.93)	-10.0	10.00	60.0	1.06 (0.40) [0.35, 1.92]
		SoC	13	11 (84.6)	-5.91 (17.60)	-35.0	-8.00	20.0	
	Week 12	Peg	29	26 (89.7)	18.88 (20.98)	-10.0	10.50	70.0	1.25 (0.40) [0.54, 2.12]
		SoC	13	11 (84.6)	-6.00 (15.03)	-36.0	-5.00	20.0	
	Week 16	Peg	29	25 (86.2)	17.04 (23.29)	-15.0	20.00	60.0	0.86 (0.38) [0.17, 1.66]
		SoC	13	12 (92.3)	-1.42 (15.08)	-37.0	0.00	20.0	
	Week 20	Peg	29	27 (93.1)	20.33 (21.79)	-15.0	20.00	70.0	1.16 (0.40) [0.45, 2.01]
		SoC	13	11 (84.6)	-3.55 (15.23)	-40.0	0.00	20.0	
	Week 26	Peg	29	23 (79.3)	21.65 (22.38)	-25.0	21.00	60.0	1.19 (0.42) [0.45, 2.11]
		SoC	13	10 (76.9)	-3.60 (15.65)	-40.0	-0.50	20.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SIHC: Change from baseline in LASA Activity Level (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in LASA Activity Level (censored)	Week 4	Peg	6	5 (83.3)	6.60 (28.25)	-17.0	-5.00	55.0	0.56 (0.82) [-0.85, 2.41]
		SoC	5	4 (80.0)	-7.50 (9.57)	-20.0	-5.00	0.0	
	Week 8	Peg	6	5 (83.3)	18.00 (21.05)	5.0	9.00	55.0	1.12 (0.89) [-0.20, 3.31]
		SoC	5	4 (80.0)	-2.50 (5.00)	-10.0	0.00	0.0	
	Week 12	Peg	6	5 (83.3)	-10.80 (38.45)	-75.0	-5.00	25.0	-0.18 (0.80) [-1.84, 1.35]
		SoC	5	4 (80.0)	-5.00 (5.77)	-10.0	-5.00	0.0	
	Week 16	Peg	6	5 (83.3)	-0.40 (47.34)	-75.0	8.00	55.0	0.17 (0.80) [-1.35, 1.83]
		SoC	5	4 (80.0)	-7.50 (9.57)	-20.0	-5.00	0.0	
	Week 20	Peg	6	5 (83.3)	18.00 (22.80)	-5.0	15.00	55.0	1.16 (0.90) [-0.16, 3.37]
		SoC	5	4 (80.0)	-5.00 (5.77)	-10.0	-5.00	0.0	
	Week 26	Peg	6	4 (66.7)	21.75 (22.82)	-7.0	24.50	45.0	1.07 (1.41) [-0.74, 4.60]
		SoC	5	2 (40.0)	-5.00 (7.07)	-10.0	-5.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SICD: Change from baseline in LASA Activity Level (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.1409

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SICD: Change from baseline in LASA Activity Level (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Male	Peg	Week 4	19	16 (84.2)	11.00 (5.17)	(0.21, 21.79)	23.45 (9.01)	(4.66, 42.25)	0.0170	*
		Week 8	19	15 (78.9)	13.52 (4.82)	(3.42, 23.61)	25.69 (8.31)	(8.31, 43.08)	0.0060	*
		Week 12	19	17 (89.5)	9.86 (5.23)	(-1.00, 20.73)	24.90 (9.38)	(5.41, 44.40)	0.0148	*
		Week 16	19	16 (84.2)	8.85 (6.21)	(-4.06, 21.75)	17.16 (10.53)	(-4.75, 39.07)	0.1182	
		Week 20	19	17 (89.5)	17.68 (3.85)	(9.67, 25.68)	31.12 (6.89)	(16.80, 45.45)	0.0002	*
		Week 26	19	14 (73.7)	21.38 (4.22)	(12.43, 30.32)	36.42 (7.80)	(19.89, 52.96)	0.0003	*
	SoC	Week 4	10	8 (80.0)	-12.45 (7.40)	(-27.89, 2.99)				
		Week 8	10	8 (80.0)	-12.18 (6.73)	(-26.25, 1.90)				
		Week 12	10	8 (80.0)	-15.04 (7.74)	(-31.13, 1.05)				
		Week 16	10	9 (90.0)	-8.31 (8.47)	(-25.93, 9.31)				
		Week 20	10	8 (80.0)	-13.45 (5.69)	(-25.28, -1.61)				
		Week 26	10	6 (60.0)	-15.05 (6.49)	(-28.81, -1.29)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SICD: Change from baseline in LASA Activity Level (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Female	Peg	Week 4	16	15 (93.8)	14.36 (4.29)	(5.34, 23.38)	11.70 (9.18)	(-7.59, 30.99)	0.2187	
		Week 8	16	14 (87.5)	17.89 (4.80)	(7.75, 28.03)	12.60 (10.27)	(-9.08, 34.28)	0.2367	
		Week 12	16	14 (87.5)	21.45 (4.49)	(11.98, 30.93)	19.63 (9.60)	(-0.64, 39.89)	0.0568	
		Week 16	16	14 (87.5)	21.09 (5.07)	(10.40, 31.79)	21.01 (10.84)	(-1.86, 43.88)	0.0694	
		Week 20	16	15 (93.8)	23.48 (4.47)	(14.09, 32.87)	20.79 (9.56)	(0.71, 40.87)	0.0432	*
		Week 26	16	13 (81.3)	23.30 (5.51)	(11.56, 35.04)	22.26 (12.06)	(-3.44, 47.96)	0.0847	
	SoC	Week 4	8	7 (87.5)	2.66 (7.52)	(-13.14, 18.46)				
		Week 8	8	7 (87.5)	5.29 (8.14)	(-11.89, 22.46)				
		Week 12	8	7 (87.5)	1.82 (7.61)	(-14.23, 17.88)				
		Week 16	8	7 (87.5)	0.08 (8.59)	(-18.04, 18.20)				
		Week 20	8	7 (87.5)	2.69 (7.83)	(-13.76, 19.14)				
		Week 26	8	6 (75.0)	1.04 (9.73)	(-19.70, 21.77)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SIHD: Change from baseline in LASA Activity Level (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Activity Level (censored)	Male	Baseline	Peg	19	19 (100.0)	64.84 (19.09)	30.0	70.00	90.0	
			SoC	10	9 (90.0)	61.11 (14.31)	45.0	60.00	90.0	
		Week 4	Peg	19	16 (84.2)	72.00 (21.26)	20.0	74.50	100.0	
			SoC	10	9 (90.0)	49.67 (17.73)	25.0	50.00	75.0	
		Week 8	Peg	19	15 (78.9)	76.07 (19.39)	30.0	80.00	100.0	
			SoC	10	9 (90.0)	50.89 (22.32)	20.0	40.00	85.0	
		Week 12	Peg	19	17 (89.5)	71.94 (25.42)	10.0	70.00	100.0	
			SoC	10	9 (90.0)	48.56 (14.17)	34.0	45.00	80.0	
		Week 16	Peg	19	16 (84.2)	72.81 (25.72)	10.0	80.00	100.0	
			SoC	10	10 (100.0)	56.10 (22.51)	33.0	50.00	100.0	
		Week 20	Peg	19	17 (89.5)	79.59 (16.48)	50.0	75.00	100.0	
			SoC	10	9 (90.0)	50.89 (16.34)	30.0	50.00	80.0	
		Week 26	Peg	19	14 (73.7)	81.36 (17.47)	50.0	86.00	100.0	
			SoC	10	7 (70.0)	48.29 (16.95)	30.0	40.00	80.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SIHD: Change from baseline in LASA Activity Level (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Activity Level (censored)	Female	Baseline	Peg	16	16 (100.0)	53.69 (20.14)	17.0	55.50	90.0	
			SoC	8	7 (87.5)	69.29 (16.94)	50.0	70.00	95.0	
		Week 4	Peg	16	15 (93.8)	71.67 (19.97)	10.0	75.00	90.0	
			SoC	8	8 (100.0)	71.50 (16.41)	50.0	71.00	100.0	
		Week 8	Peg	16	14 (87.5)	75.21 (19.51)	20.0	80.00	100.0	
			SoC	8	7 (87.5)	70.29 (16.35)	50.0	70.00	90.0	
		Week 12	Peg	16	14 (87.5)	77.00 (21.26)	10.0	80.00	100.0	
			SoC	8	7 (87.5)	71.71 (16.75)	50.0	72.00	90.0	
		Week 16	Peg	16	14 (87.5)	76.36 (21.37)	20.0	85.00	94.0	
			SoC	8	7 (87.5)	72.43 (17.74)	50.0	72.00	95.0	
		Week 20	Peg	16	15 (93.8)	80.40 (20.53)	10.0	90.00	95.0	
			SoC	8	8 (100.0)	73.50 (16.83)	50.0	76.00	96.0	
		Week 26	Peg	16	13 (81.3)	78.54 (24.14)	10.0	80.00	100.0	
			SoC	8	6 (75.0)	76.00 (15.90)	50.0	76.00	94.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SIHD: Change from baseline in LASA Activity Level (censored) by sex
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Male in LASA Activity Level (censored)	Week 4	Peg	19	16 (84.2)	9.38 (26.75)	-25.0	3.50	70.0	0.79 (0.47) [-0.06, 1.81]
		SoC	10	8 (80.0)	-10.38 (16.66)	-45.0	-8.00	10.0	
	Week 8	Peg	19	15 (78.9)	12.53 (20.56)	-10.0	6.00	60.0	1.13 (0.50) [0.27, 2.23]
		SoC	10	8 (80.0)	-10.25 (17.13)	-35.0	-11.00	20.0	
	Week 12	Peg	19	17 (89.5)	7.71 (30.38)	-75.0	10.00	70.0	0.76 (0.47) [-0.08, 1.76]
		SoC	10	8 (80.0)	-12.88 (11.58)	-36.0	-11.00	0.0	
	Week 16	Peg	19	16 (84.2)	7.94 (31.47)	-75.0	9.00	60.0	0.56 (0.45) [-0.26, 1.49]
		SoC	10	9 (90.0)	-7.67 (15.55)	-37.0	-10.00	15.0	
	Week 20	Peg	19	17 (89.5)	16.18 (22.83)	-15.0	15.00	70.0	1.25 (0.49) [0.41, 2.34]
		SoC	10	8 (80.0)	-10.25 (13.38)	-40.0	-10.00	5.0	
	Week 26	Peg	19	14 (73.7)	19.71 (22.15)	-9.0	17.50	60.0	1.45 (0.58) [0.49, 2.78]
		SoC	10	6 (60.0)	-11.17 (14.97)	-40.0	-7.50	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SIHD: Change from baseline in LASA Activity Level (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in LASA Activity Level (censored)	Female	Week 4	Peg	16	15 (93.8)	15.53 (19.34)	-25.0	15.00	55.0	0.85 (0.50) [-0.05, 1.94]
			SoC	8	7 (87.5)	1.00 (5.51)	-8.0	0.00	10.0	
		Week 8	Peg	16	14 (87.5)	20.07 (20.65)	-10.0	15.00	55.0	1.01 (0.52) [0.11, 2.17]
			SoC	8	7 (87.5)	1.00 (10.66)	-10.0	0.00	20.0	
		Week 12	Peg	16	14 (87.5)	21.86 (18.20)	-10.0	22.50	59.0	1.17 (0.53) [0.26, 2.36]
			SoC	8	7 (87.5)	2.43 (9.55)	-8.0	0.00	20.0	
		Week 16	Peg	16	14 (87.5)	21.21 (23.32)	-15.0	22.00	59.0	0.87 (0.51) [-0.04, 1.99]
			SoC	8	7 (87.5)	3.14 (9.08)	-8.0	0.00	20.0	
		Week 20	Peg	16	15 (93.8)	24.27 (19.97)	-10.0	29.00	59.0	1.16 (0.52) [0.26, 2.32]
			SoC	8	7 (87.5)	3.29 (9.03)	-8.0	0.00	20.0	
		Week 26	Peg	16	13 (81.3)	23.77 (22.52)	-25.0	30.00	59.0	0.98 (0.56) [0.02, 2.22]
			SoC	8	6 (75.0)	3.50 (9.91)	-8.0	0.00	20.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SICB: Change from baseline in LASA Activity Level (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.0507

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SICB: Change from baseline in LASA Activity Level (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Latin America	Peg	Week 4	12	11 (91.7)	30.85 (3.28)	(23.42, 38.27)	38.81 (8.35)	(19.91, 57.70)	0.0012	*
		Week 8	12	10 (83.3)	33.12 (3.07)	(26.03, 40.21)	41.24 (7.54)	(23.85, 58.63)	0.0006	*
		Week 12	12	11 (91.7)	25.13 (6.09)	(11.35, 38.91)	34.23 (15.50)	(-0.83, 69.29)	0.0546	
		Week 16	12	10 (83.3)	26.88 (5.82)	(13.45, 40.31)	36.77 (14.28)	(3.84, 69.70)	0.0329	*
		Week 20	12	10 (83.3)	38.13 (3.79)	(29.38, 46.88)	43.76 (9.29)	(22.33, 65.19)	0.0015	*
		Week 26	12	9 (75.0)	40.69 (3.18)	(33.19, 48.20)	44.57 (7.44)	(26.99, 62.16)	0.0005	*
	SoC	Week 4	2	2 (100.0)	-7.96 (7.68)	(-25.33, 9.41)				
		Week 8	2	2 (100.0)	-8.12 (6.88)	(-23.99, 7.75)				
		Week 12	2	2 (100.0)	-9.09 (14.25)	(-41.32, 23.13)				
		Week 16	2	2 (100.0)	-9.89 (13.03)	(-39.95, 20.16)				
		Week 20	2	2 (100.0)	-5.64 (8.48)	(-25.20, 13.93)				
		Week 26	2	2 (100.0)	-3.88 (6.72)	(-19.76, 12.00)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SICB: Change from baseline in LASA Activity Level (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Asia Pacific Peg	Peg	Week 4	23	20 (87.0)	3.23 (3.59)	(-4.11, 10.56)	9.77 (5.89)	(-2.27, 21.81)	0.1076	
		Week 8	23	19 (82.6)	7.62 (3.49)	(0.46, 14.77)	12.67 (5.66)	(1.08, 24.26)	0.0332	*
		Week 12	23	20 (87.0)	9.70 (3.11)	(3.33, 16.07)	17.02 (5.11)	(6.57, 27.48)	0.0024	*
		Week 16	23	20 (87.0)	8.89 (3.47)	(1.80, 15.97)	12.44 (5.51)	(1.20, 23.68)	0.0313	*
		Week 20	23	22 (95.7)	12.25 (3.11)	(5.91, 18.58)	17.07 (5.24)	(6.38, 27.76)	0.0027	*
	Week 26	23	18 (78.3)	12.59 (3.91)	(4.53, 20.65)	19.33 (6.93)	(5.03, 33.63)	0.0102	*	
	SoC	Week 4	16	13 (81.3)	-6.54 (4.53)	(-15.80, 2.72)				
		Week 8	16	13 (81.3)	-5.05 (4.27)	(-13.79, 3.69)				
		Week 12	16	13 (81.3)	-7.33 (3.93)	(-15.37, 0.72)				
		Week 16	16	14 (87.5)	-3.55 (4.17)	(-12.07, 4.96)				
Week 20		16	13 (81.3)	-4.82 (4.11)	(-13.21, 3.57)					
Week 26	16	10 (62.5)	-6.73 (5.59)	(-18.27, 4.81)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SIHB: Change from baseline in LASA Activity Level (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Activity Level (censored)	Latin America	Baseline	Peg	12	12 (100.0)	49.33 (21.87)	17.0	49.50	85.0	
			SoC	2	2 (100.0)	50.00 (0.00)	50.0	50.00	50.0	
		Week 4	Peg	12	11 (91.7)	82.73 (9.95)	70.0	85.00	100.0	
			SoC	2	2 (100.0)	44.00 (8.49)	38.0	44.00	50.0	
		Week 8	Peg	12	10 (83.3)	85.30 (8.74)	70.0	85.00	100.0	
			SoC	2	2 (100.0)	44.00 (8.49)	38.0	44.00	50.0	
		Week 12	Peg	12	11 (91.7)	75.91 (25.20)	10.0	80.00	100.0	
			SoC	2	2 (100.0)	44.00 (8.49)	38.0	44.00	50.0	
		Week 16	Peg	12	10 (83.3)	78.70 (25.32)	10.0	90.00	97.0	
			SoC	2	2 (100.0)	44.00 (8.49)	38.0	44.00	50.0	
		Week 20	Peg	12	10 (83.3)	87.80 (11.13)	70.0	90.00	100.0	
			SoC	2	2 (100.0)	44.00 (8.49)	38.0	44.00	50.0	
		Week 26	Peg	12	9 (75.0)	87.67 (9.57)	70.0	90.00	100.0	
			SoC	2	2 (100.0)	44.00 (8.49)	38.0	44.00	50.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SIHB: Change from baseline in LASA Activity Level (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Activity Level (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	65.17 (17.17)	20.0	70.00	90.0	
			SoC	16	14 (87.5)	66.79 (15.52)	45.0	67.50	95.0	
		Week 4	Peg	23	20 (87.0)	65.85 (22.18)	10.0	70.00	100.0	
			SoC	16	15 (93.8)	62.07 (20.30)	25.0	70.00	100.0	
		Week 8	Peg	23	19 (82.6)	70.58 (21.22)	20.0	70.00	100.0	
			SoC	16	14 (87.5)	61.57 (22.29)	20.0	70.00	90.0	
		Week 12	Peg	23	20 (87.0)	73.30 (22.96)	10.0	80.00	100.0	
			SoC	16	14 (87.5)	60.79 (19.29)	34.0	55.00	90.0	
		Week 16	Peg	23	20 (87.0)	72.35 (22.84)	20.0	80.00	100.0	
			SoC	16	15 (93.8)	65.33 (21.83)	33.0	70.00	100.0	
		Week 20	Peg	23	22 (95.7)	76.41 (19.82)	10.0	80.00	100.0	
			SoC	16	15 (93.8)	63.87 (19.85)	30.0	70.00	96.0	
		Week 26	Peg	23	18 (78.3)	76.17 (23.63)	10.0	80.00	100.0	
			SoC	16	11 (68.8)	64.18 (21.70)	30.0	70.00	94.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SIHB: Change from baseline in LASA Activity Level (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in LASA Activity Level (censored)	Latin America	Week 4	Peg	12	11 (91.7)	30.45 (22.95)	1.0	25.00	70.0	1.54 (0.94) [0.03, 3.73]
			SoC	2	2 (100.0)	-6.00 (8.49)	-12.0	-6.00	0.0	
		Week 8	Peg	12	10 (83.3)	32.70 (22.64)	-5.0	36.50	60.0	1.65 (0.98) [0.13, 3.99]
			SoC	2	2 (100.0)	-6.00 (8.49)	-12.0	-6.00	0.0	
		Week 12	Peg	12	11 (91.7)	23.64 (39.12)	-75.0	29.00	70.0	0.74 (0.87) [-0.82, 2.63]
			SoC	2	2 (100.0)	-6.00 (8.49)	-12.0	-6.00	0.0	
		Week 16	Peg	12	10 (83.3)	26.10 (42.63)	-75.0	38.50	60.0	0.73 (0.89) [-0.85, 2.68]
			SoC	2	2 (100.0)	-6.00 (8.49)	-12.0	-6.00	0.0	
		Week 20	Peg	12	10 (83.3)	38.20 (23.55)	-5.0	39.00	70.0	1.81 (1.00) [0.30, 4.23]
			SoC	2	2 (100.0)	-6.00 (8.49)	-12.0	-6.00	0.0	
		Week 26	Peg	12	9 (75.0)	40.89 (16.91)	15.0	40.00	60.0	2.65 (1.19) [1.07, 5.69]
			SoC	2	2 (100.0)	-6.00 (8.49)	-12.0	-6.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SIHB: Change from baseline in LASA Activity Level (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in LASA Activity Level (censored)	Asia Pacific	Week 4	Peg	23	20 (87.0)	2.40 (16.88)	-25.0	5.50	30.0	0.45 (0.37) [-0.26, 1.21]
			SoC	16	13 (81.3)	-4.92 (14.53)	-45.0	0.00	10.0	
		Week 8	Peg	23	19 (82.6)	7.47 (13.09)	-10.0	6.00	40.0	0.83 (0.39) [0.13, 1.67]
			SoC	16	13 (81.3)	-4.85 (16.16)	-35.0	-5.00	20.0	
		Week 12	Peg	23	20 (87.0)	8.85 (14.06)	-10.0	10.00	40.0	1.02 (0.39) [0.32, 1.86]
			SoC	16	13 (81.3)	-5.69 (13.81)	-36.0	-5.00	20.0	
		Week 16	Peg	23	20 (87.0)	8.15 (15.80)	-15.0	10.00	40.0	0.68 (0.37) [-0.01, 1.46]
			SoC	16	14 (87.5)	-2.50 (14.66)	-37.0	0.00	20.0	
		Week 20	Peg	23	22 (95.7)	11.68 (14.84)	-15.0	10.00	40.0	1.03 (0.39) [0.34, 1.86]
			SoC	16	13 (81.3)	-3.62 (13.98)	-40.0	0.00	20.0	
		Week 26	Peg	23	18 (78.3)	12.06 (17.67)	-25.0	10.00	34.0	0.88 (0.43) [0.11, 1.81]
			SoC	16	10 (62.5)	-3.40 (15.54)	-40.0	-0.50	20.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SICA: Change from baseline in LASA Activity Level (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.3936

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SICA: Change from baseline in LASA Activity Level (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 4	21	18 (85.7)	9.29 (5.24)	(-1.63, 20.21)	18.34 (11.28)	(-5.18, 41.86)	0.1195	
		Week 8	21	17 (81.0)	15.31 (4.66)	(5.55, 25.07)	23.35 (9.81)	(2.82, 43.89)	0.0279	*
		Week 12	21	18 (85.7)	9.93 (5.67)	(-1.91, 21.76)	26.26 (12.22)	(0.78, 51.75)	0.0440	*
		Week 16	21	17 (81.0)	7.97 (6.54)	(-5.66, 21.60)	12.06 (12.99)	(-15.04, 39.15)	0.3643	
		Week 20	21	18 (85.7)	19.08 (4.23)	(10.26, 27.89)	29.95 (9.12)	(10.93, 48.97)	0.0037	*
	Week 26	21	15 (71.4)	18.87 (5.42)	(7.32, 30.42)	34.24 (13.31)	(5.87, 62.61)	0.0212	*	
	SoC	Week 4	8	5 (62.5)	-9.05 (9.96)	(-29.84, 11.74)				
		Week 8	8	5 (62.5)	-8.05 (8.62)	(-26.08, 9.99)				
		Week 12	8	5 (62.5)	-16.34 (10.80)	(-38.85, 6.18)				
		Week 16	8	6 (75.0)	-4.09 (11.11)	(-27.26, 19.09)				
		Week 20	8	5 (62.5)	-10.87 (8.05)	(-27.67, 5.93)				
Week 26		8	3 (37.5)	-15.37 (12.14)	(-41.25, 10.52)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SICA: Change from baseline in LASA Activity Level (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	15.39 (4.09)	(6.85, 23.92)	16.89 (6.21)	(3.93, 29.85)	0.0133	*
		Week 8	14	12 (85.7)	16.29 (4.85)	(6.14, 26.44)	18.44 (7.20)	(3.36, 33.52)	0.0192	*
		Week 12	14	13 (92.9)	18.93 (4.27)	(10.01, 27.84)	18.13 (6.48)	(4.62, 31.65)	0.0111	*
		Week 16	14	13 (92.9)	19.86 (4.26)	(10.99, 28.74)	19.09 (6.45)	(5.62, 32.55)	0.0078	*
		Week 20	14	14 (100.0)	20.26 (4.34)	(11.23, 29.28)	19.52 (6.72)	(5.54, 33.50)	0.0085	*
		Week 26	14	12 (85.7)	24.16 (4.35)	(15.02, 33.30)	22.81 (6.66)	(8.82, 36.80)	0.0030	*
	SoC	Week 4	10	10 (100.0)	-1.50 (4.67)	(-11.24, 8.24)				
		Week 8	10	10 (100.0)	-2.15 (5.31)	(-13.27, 8.97)				
		Week 12	10	10 (100.0)	0.79 (4.87)	(-9.37, 10.96)				
		Week 16	10	10 (100.0)	0.78 (4.85)	(-9.35, 10.90)				
		Week 20	10	10 (100.0)	0.74 (5.13)	(-9.94, 11.41)				
		Week 26	10	9 (90.0)	1.35 (5.03)	(-9.22, 11.91)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SIHA: Change from baseline in LASA Activity Level (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Activity Level (censored)	< 4	Baseline	Peg	21	21 (100.0)	57.43 (22.29)	17.0	60.00	90.0	
			SoC	8	6 (75.0)	67.50 (13.32)	50.0	67.50	90.0	
		Week 4	Peg	21	18 (85.7)	68.22 (23.46)	10.0	72.50	91.0	
			SoC	8	7 (87.5)	58.29 (20.06)	25.0	60.00	80.0	
		Week 8	Peg	21	17 (81.0)	74.53 (21.98)	20.0	80.00	100.0	
			SoC	8	6 (75.0)	58.00 (22.85)	35.0	55.00	85.0	
		Week 12	Peg	21	18 (85.7)	68.83 (26.96)	10.0	79.00	100.0	
			SoC	8	6 (75.0)	50.33 (17.32)	34.0	45.00	80.0	
		Week 16	Peg	21	17 (81.0)	68.24 (26.86)	10.0	80.00	90.0	
			SoC	8	7 (87.5)	61.57 (25.15)	33.0	50.00	100.0	
		Week 20	Peg	21	18 (85.7)	77.28 (19.85)	10.0	80.00	100.0	
			SoC	8	7 (87.5)	58.29 (19.85)	30.0	60.00	80.0	
		Week 26	Peg	21	15 (71.4)	74.93 (23.37)	10.0	80.00	100.0	
			SoC	8	4 (50.0)	52.00 (22.57)	30.0	49.00	80.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SIHA: Change from baseline in LASA Activity Level (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Activity Level (censored)	>= 4	Baseline	Peg	14	14 (100.0)	63.21 (16.44)	30.0	60.00	90.0	
			SoC	10	10 (100.0)	63.00 (17.19)	45.0	55.00	95.0	
		Week 4	Peg	14	13 (92.9)	76.85 (14.26)	50.0	80.00	100.0	
			SoC	10	10 (100.0)	61.10 (21.01)	30.0	60.00	100.0	
		Week 8	Peg	14	12 (85.7)	77.25 (14.88)	50.0	80.00	100.0	
			SoC	10	10 (100.0)	60.20 (22.22)	20.0	60.00	90.0	
		Week 12	Peg	14	13 (92.9)	81.69 (15.30)	50.0	80.00	100.0	
			SoC	10	10 (100.0)	63.70 (18.95)	40.0	60.00	90.0	
		Week 16	Peg	14	13 (92.9)	82.62 (15.51)	50.0	90.00	100.0	
			SoC	10	10 (100.0)	63.70 (20.36)	40.0	60.00	95.0	
		Week 20	Peg	14	14 (100.0)	83.43 (15.83)	50.0	90.00	100.0	
			SoC	10	10 (100.0)	63.80 (20.54)	40.0	60.00	96.0	
		Week 26	Peg	14	12 (85.7)	86.33 (15.09)	50.0	90.00	100.0	
			SoC	10	9 (90.0)	65.11 (20.81)	40.0	70.00	94.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SIHA: Change from baseline in LASA Activity Level (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in LASA Activity Level (censored)	Week 4	Peg	21	18 (85.7)	9.94 (25.97)	-25.0	8.00	60.0	0.82 (0.55) [-0.17, 2.00]
		SoC	8	5 (62.5)	-11.40 (20.73)	-45.0	-10.00	10.0	
	Week 8	Peg	21	17 (81.0)	15.71 (20.49)	-10.0	8.00	55.0	1.18 (0.57) [0.18, 2.44]
		SoC	8	5 (62.5)	-9.40 (20.78)	-35.0	-12.00	20.0	
	Week 12	Peg	21	18 (85.7)	10.56 (28.92)	-75.0	10.00	59.0	1.06 (0.56) [0.07, 2.29]
		SoC	8	5 (62.5)	-18.60 (10.43)	-36.0	-15.00	-10.0	
	Week 16	Peg	21	17 (81.0)	9.71 (32.84)	-75.0	10.00	60.0	0.60 (0.51) [-0.34, 1.67]
		SoC	8	6 (75.0)	-9.00 (19.25)	-37.0	-11.00	15.0	
	Week 20	Peg	21	18 (85.7)	19.78 (21.73)	-15.0	20.00	60.0	1.54 (0.59) [0.54, 2.87]
		SoC	8	5 (62.5)	-13.40 (16.36)	-40.0	-10.00	5.0	
	Week 26	Peg	21	15 (71.4)	19.27 (23.81)	-25.0	20.00	60.0	1.49 (0.74) [0.25, 3.16]
		SoC	8	3 (37.5)	-17.33 (20.53)	-40.0	-12.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.

95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.

Source Data: apro, created on: 18SEP2023

Table PT1LAWC_SIHA: Change from baseline in LASA Activity Level (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in LASA Activity Level (censored)	Week 4	Peg	14	13 (92.9)	15.69 (19.47)	-6.0	10.00	70.0	1.08 (0.48) [0.26, 2.15]
		SoC	10	10 (100.0)	-1.90 (8.09)	-20.0	0.00	10.0	
	Week 8	Peg	14	12 (85.7)	16.83 (21.62)	-10.0	10.00	60.0	1.05 (0.49) [0.21, 2.13]
		SoC	10	10 (100.0)	-2.80 (12.18)	-25.0	-2.50	20.0	
	Week 12	Peg	14	13 (92.9)	19.00 (22.08)	-9.0	11.00	70.0	1.00 (0.47) [0.18, 2.04]
		SoC	10	10 (100.0)	0.70 (8.72)	-10.0	0.00	20.0	
	Week 16	Peg	14	13 (92.9)	19.92 (20.86)	-9.0	20.00	60.0	1.10 (0.48) [0.28, 2.17]
		SoC	10	10 (100.0)	0.70 (8.72)	-10.0	0.00	20.0	
	Week 20	Peg	14	14 (100.0)	20.21 (22.22)	-9.0	15.00	70.0	1.04 (0.47) [0.23, 2.07]
		SoC	10	10 (100.0)	0.80 (8.72)	-10.0	0.00	20.0	
	Week 26	Peg	14	12 (85.7)	24.67 (20.11)	-9.0	30.00	60.0	1.40 (0.53) [0.53, 2.62]
		SoC	10	9 (90.0)	0.67 (9.26)	-10.0	0.00	20.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SICC: Change from baseline in LASA Daily Activities (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.2474

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SICC: Change from baseline in LASA Daily Activities (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
< 60	Peg	Week 4	29	26 (89.7)	12.64 (3.59)	(5.35, 19.94)	14.11 (6.65)	(0.58, 27.65)	0.0415	*
		Week 8	29	24 (82.8)	15.60 (3.69)	(8.06, 23.13)	18.95 (6.67)	(5.35, 32.54)	0.0079	*
		Week 12	29	26 (89.7)	17.29 (3.78)	(9.61, 24.98)	20.11 (7.00)	(5.86, 34.36)	0.0071	*
		Week 16	29	25 (86.2)	16.82 (3.83)	(9.02, 24.61)	13.37 (6.76)	(-0.39, 27.12)	0.0564	
		Week 20	29	27 (93.1)	18.23 (3.32)	(11.49, 24.97)	20.62 (6.23)	(7.96, 33.29)	0.0022	*
		Week 26	29	23 (79.3)	19.34 (3.88)	(11.41, 27.27)	20.38 (7.22)	(5.61, 35.15)	0.0085	*
	SoC	Week 4	13	11 (84.6)	-1.47 (5.53)	(-12.72, 9.78)				
		Week 8	13	11 (84.6)	-3.35 (5.47)	(-14.50, 7.80)				
		Week 12	13	11 (84.6)	-2.82 (5.82)	(-14.66, 9.03)				
		Week 16	13	12 (92.3)	3.45 (5.53)	(-7.80, 14.70)				
		Week 20	13	11 (84.6)	-2.39 (5.23)	(-13.02, 8.24)				
		Week 26	13	10 (76.9)	-1.04 (5.99)	(-13.30, 11.22)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SICC: Change from baseline in LASA Daily Activities (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	4.81 (7.09)	(-13.42, 23.03)	13.17 (11.27)	(-15.79, 42.14)	0.2950	
		Week 8	6	5 (83.3)	12.03 (6.05)	(-3.51, 27.58)	21.77 (9.61)	(-2.92, 46.47)	0.0728	
		Week 12	6	5 (83.3)	-18.15 (15.78)	(-58.71, 22.41)	0.95 (25.07)	(-63.49, 65.40)	0.9711	
		Week 16	6	5 (83.3)	0.06 (13.84)	(-35.50, 35.63)	15.95 (21.78)	(-40.04, 71.94)	0.4967	
		Week 20	6	5 (83.3)	13.20 (5.40)	(-0.68, 27.08)	23.36 (8.50)	(1.51, 45.21)	0.0404	*
		Week 26	6	4 (66.7)	33.26 (10.81)	(-13.27, 79.79)	53.70 (15.27)	(-12.00, 119.39)	0.0722	
	SoC	Week 4	5	4 (80.0)	-8.37 (6.42)	(-24.88, 8.15)				
		Week 8	5	4 (80.0)	-9.74 (5.48)	(-23.82, 4.35)				
		Week 12	5	4 (80.0)	-19.11 (14.30)	(-55.85, 17.64)				
		Week 16	5	4 (80.0)	-15.89 (15.93)	(-56.85, 25.06)				
		Week 20	5	4 (80.0)	-10.16 (6.22)	(-26.15, 5.82)				
		Week 26	5	2 (40.0)	-20.43 (9.46)	(-61.15, 20.28)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SIHC: Change from baseline in LASA Daily Activities (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Daily Activities (censored)	< 60	Baseline	Peg	29	29 (100.0)	60.34 (23.79)	10.0	66.00	90.0	
			SoC	13	12 (92.3)	64.00 (16.24)	40.0	60.00	95.0	
		Week 4	Peg	29	26 (89.7)	72.58 (20.62)	2.0	75.00	100.0	
			SoC	13	12 (92.3)	63.58 (21.64)	27.0	67.50	92.0	
		Week 8	Peg	29	24 (82.8)	76.04 (19.62)	20.0	80.00	100.0	
			SoC	13	11 (84.6)	60.18 (23.84)	20.0	65.00	90.0	
		Week 12	Peg	29	26 (89.7)	77.85 (22.22)	10.0	80.00	100.0	
			SoC	13	11 (84.6)	61.09 (21.89)	35.0	50.00	100.0	
		Week 16	Peg	29	25 (86.2)	78.60 (20.79)	20.0	90.00	100.0	
			SoC	13	12 (92.3)	67.92 (24.13)	34.0	69.00	100.0	
		Week 20	Peg	29	27 (93.1)	79.63 (19.44)	10.0	80.00	100.0	
			SoC	13	12 (92.3)	63.42 (21.30)	30.0	67.50	95.0	
		Week 26	Peg	29	23 (79.3)	79.26 (22.77)	10.0	87.00	100.0	
			SoC	13	10 (76.9)	62.70 (21.47)	32.0	62.50	94.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SIHC: Change from baseline in LASA Daily Activities (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Daily Activities (censored)	>= 60	Baseline	Peg	6	6 (100.0)	72.00 (18.20)	50.0	73.50	95.0	
			SoC	5	4 (80.0)	57.50 (15.00)	50.0	50.00	80.0	
	Week 4	Peg	6	5 (83.3)	79.60 (19.36)	50.0	86.00	100.0		
		SoC	5	5 (100.0)	55.00 (18.03)	30.0	50.00	75.0		
	Week 8	Peg	6	5 (83.3)	86.40 (13.69)	65.0	90.00	100.0		
		SoC	5	5 (100.0)	54.80 (17.75)	30.0	50.00	74.0		
	Week 12	Peg	6	5 (83.3)	54.80 (25.32)	10.0	66.00	70.0		
		SoC	5	5 (100.0)	52.80 (16.10)	30.0	50.00	74.0		
	Week 16	Peg	6	5 (83.3)	63.60 (32.78)	10.0	68.00	90.0		
		SoC	5	5 (100.0)	52.80 (16.10)	30.0	50.00	74.0		
	Week 20	Peg	6	5 (83.3)	80.40 (19.77)	50.0	90.00	100.0		
		SoC	5	5 (100.0)	52.80 (16.10)	30.0	50.00	74.0		
	Week 26	Peg	6	4 (66.7)	87.00 (12.99)	69.0	89.50	100.0		
		SoC	5	3 (60.0)	51.33 (22.03)	30.0	50.00	74.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SIHC: Change from baseline in LASA Daily Activities (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in LASA Daily Activities (censored)	Week 4	Peg	29	26 (89.7)	11.96 (23.32)	-20.0	9.50	70.0	0.55 (0.38) [-0.16, 1.33]
		SoC	13	11 (84.6)	-0.45 (18.54)	-43.0	0.00	30.0	
	Week 8	Peg	29	24 (82.8)	14.92 (21.94)	-20.0	10.00	60.0	0.80 (0.39) [0.09, 1.62]
		SoC	13	11 (84.6)	-2.36 (19.20)	-34.0	0.00	30.0	
	Week 12	Peg	29	26 (89.7)	16.46 (23.92)	-35.0	15.00	70.0	0.77 (0.38) [0.07, 1.57]
		SoC	13	11 (84.6)	-1.45 (19.59)	-35.0	0.00	30.0	
	Week 16	Peg	29	25 (86.2)	16.32 (24.83)	-30.0	10.00	69.0	0.53 (0.37) [-0.16, 1.28]
		SoC	13	12 (92.3)	3.92 (18.61)	-36.0	4.00	30.0	
	Week 20	Peg	29	27 (93.1)	17.52 (23.31)	-10.0	10.00	70.0	0.80 (0.38) [0.10, 1.60]
		SoC	13	11 (84.6)	-0.64 (18.69)	-40.0	0.00	30.0	
	Week 26	Peg	29	23 (79.3)	18.57 (23.09)	-20.0	10.00	69.0	0.77 (0.41) [0.02, 1.62]
		SoC	13	10 (76.9)	1.40 (18.48)	-38.0	0.00	30.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SIHC: Change from baseline in LASA Daily Activities (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in LASA Daily Activities (censored)	Week 4	Peg	6	5 (83.3)	3.20 (8.61)	-5.0	3.00	15.0	1.05 (0.88) [-0.28, 3.19]
		SoC	5	4 (80.0)	-7.50 (9.57)	-20.0	-5.00	0.0	
	Week 8	Peg	6	5 (83.3)	10.00 (7.04)	0.0	10.00	18.0	1.89 (1.05) [0.56, 4.64]
		SoC	5	4 (80.0)	-7.50 (9.57)	-20.0	-5.00	0.0	
	Week 12	Peg	6	5 (83.3)	-21.60 (35.52)	-75.0	-17.00	15.0	-0.37 (0.81) [-2.12, 1.09]
		SoC	5	4 (80.0)	-10.00 (11.55)	-20.0	-10.00	0.0	
	Week 16	Peg	6	5 (83.3)	-6.20 (42.13)	-75.0	4.00	40.0	0.10 (0.79) [-1.44, 1.73]
		SoC	5	4 (80.0)	-10.00 (11.55)	-20.0	-10.00	0.0	
	Week 20	Peg	6	5 (83.3)	10.60 (18.56)	-5.0	8.00	40.0	1.15 (0.90) [-0.17, 3.35]
		SoC	5	4 (80.0)	-10.00 (11.55)	-20.0	-10.00	0.0	
	Week 26	Peg	6	4 (66.7)	12.25 (16.64)	-5.0	10.00	34.0	1.11 (1.43) [-0.69, 4.69]
		SoC	5	2 (40.0)	-10.00 (14.14)	-20.0	-10.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SICD: Change from baseline in LASA Daily Activities (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Sex	Peg	Overall								0.0636

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SICD: Change from baseline in LASA Daily Activities (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Male	Peg	Week 4	19	16 (84.2)	15.81 (4.78)	(5.83, 25.79)	26.76 (8.28)	(9.49, 44.02)	0.0042	*
		Week 8	19	15 (78.9)	15.06 (4.53)	(5.58, 24.54)	28.99 (7.75)	(12.78, 45.21)	0.0014	*
		Week 12	19	17 (89.5)	9.82 (5.41)	(-1.44, 21.07)	25.71 (9.64)	(5.67, 45.75)	0.0144	*
		Week 16	19	16 (84.2)	11.73 (6.34)	(-1.45, 24.91)	19.34 (10.71)	(-2.93, 41.62)	0.0853	
		Week 20	19	17 (89.5)	18.23 (4.07)	(9.77, 26.68)	33.09 (7.22)	(18.08, 48.11)	0.0002	*
	Week 26	19	14 (73.7)	20.68 (4.59)	(10.96, 30.41)	35.61 (8.52)	(17.55, 53.67)	0.0007	*	
	SoC	Week 4	10	8 (80.0)	-10.95 (6.82)	(-25.16, 3.27)				
		Week 8	10	8 (80.0)	-13.93 (6.29)	(-27.10, -0.76)				
		Week 12	10	8 (80.0)	-15.89 (7.98)	(-32.48, 0.71)				
		Week 16	10	9 (90.0)	-7.61 (8.63)	(-25.56, 10.33)				
		Week 20	10	8 (80.0)	-14.87 (5.99)	(-27.31, -2.42)				
Week 26	10	6 (60.0)	-14.93 (7.08)	(-29.93, 0.07)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SICD: Change from baseline in LASA Daily Activities (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Female	Peg	Week 4	16	15 (93.8)	6.55 (3.97)	(-1.79, 14.88)	4.43 (8.38)	(-13.17, 22.03)	0.6032	
		Week 8	16	14 (87.5)	15.53 (4.13)	(6.82, 24.24)	16.04 (8.68)	(-2.26, 34.34)	0.0819	
		Week 12	16	14 (87.5)	14.05 (5.22)	(3.03, 25.07)	9.00 (10.98)	(-14.18, 32.17)	0.4241	
		Week 16	16	14 (87.5)	16.98 (4.68)	(7.10, 26.86)	16.99 (9.84)	(-3.78, 37.76)	0.1025	
		Week 20	16	15 (93.8)	16.78 (4.21)	(7.95, 25.62)	16.56 (8.88)	(-2.10, 35.23)	0.0787	
		Week 26	16	13 (81.3)	17.62 (5.04)	(6.88, 28.37)	14.09 (10.75)	(-8.81, 37.00)	0.2095	
	SoC	Week 4	8	7 (87.5)	2.11 (6.94)	(-12.46, 16.69)				
		Week 8	8	7 (87.5)	-0.51 (6.96)	(-15.19, 14.17)				
		Week 12	8	7 (87.5)	5.05 (8.81)	(-13.54, 23.64)				
		Week 16	8	7 (87.5)	-0.01 (7.90)	(-16.68, 16.65)				
		Week 20	8	7 (87.5)	0.22 (7.36)	(-15.24, 15.68)				
		Week 26	8	6 (75.0)	3.53 (8.83)	(-15.29, 22.36)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SIHD: Change from baseline in LASA Daily Activities (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Daily Activities (censored)	Male	Baseline	Peg	19	19 (100.0)	62.95 (19.63)	30.0	64.00	90.0	
			SoC	10	9 (90.0)	61.44 (15.21)	40.0	60.00	80.0	
		Week 4	Peg	19	16 (84.2)	76.81 (18.90)	40.0	78.50	100.0	
			SoC	10	9 (90.0)	52.00 (20.42)	27.0	50.00	85.0	
		Week 8	Peg	19	15 (78.9)	77.67 (18.60)	40.0	82.00	100.0	
			SoC	10	9 (90.0)	49.78 (21.92)	20.0	43.00	85.0	
		Week 12	Peg	19	17 (89.5)	71.53 (25.96)	10.0	70.00	100.0	
			SoC	10	9 (90.0)	47.56 (13.29)	30.0	46.00	74.0	
		Week 16	Peg	19	16 (84.2)	74.13 (25.59)	10.0	80.00	100.0	
			SoC	10	10 (100.0)	56.10 (22.38)	30.0	50.00	100.0	
		Week 20	Peg	19	17 (89.5)	79.65 (18.03)	50.0	80.00	100.0	
			SoC	10	9 (90.0)	49.11 (15.98)	30.0	45.00	74.0	
		Week 26	Peg	19	14 (73.7)	80.79 (19.04)	40.0	88.00	100.0	
			SoC	10	7 (70.0)	47.00 (15.74)	30.0	43.00	74.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SIHD: Change from baseline in LASA Daily Activities (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Daily Activities (censored)	Female	Baseline	Peg	16	16 (100.0)	61.63 (27.36)	10.0	70.00	95.0	
			SoC	8	7 (87.5)	63.57 (17.49)	50.0	60.00	95.0	
		Week 4	Peg	16	15 (93.8)	70.40 (21.81)	2.0	80.00	90.0	
			SoC	8	8 (100.0)	71.25 (16.06)	50.0	71.50	92.0	
		Week 8	Peg	16	14 (87.5)	78.00 (20.01)	20.0	80.00	100.0	
			SoC	8	7 (87.5)	69.71 (16.50)	50.0	70.00	90.0	
		Week 12	Peg	16	14 (87.5)	77.29 (21.66)	10.0	80.00	100.0	
			SoC	8	7 (87.5)	72.57 (19.10)	50.0	73.00	100.0	
		Week 16	Peg	16	14 (87.5)	78.36 (20.85)	20.0	90.00	97.0	
			SoC	8	7 (87.5)	74.00 (20.09)	50.0	73.00	100.0	
		Week 20	Peg	16	15 (93.8)	79.87 (21.03)	10.0	90.00	95.0	
			SoC	8	8 (100.0)	72.88 (16.87)	50.0	76.50	95.0	
		Week 26	Peg	16	13 (81.3)	80.00 (24.83)	10.0	90.00	100.0	
			SoC	8	6 (75.0)	75.33 (16.37)	50.0	76.50	94.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SIHD: Change from baseline in LASA Daily Activities (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in LASA Daily Activities (censored)	Male	Week 4	Peg	19	16 (84.2)	15.19 (24.00)	-20.0	10.50	70.0	1.11 (0.49) [0.26, 2.20]
			SoC	10	8 (80.0)	-10.00 (16.29)	-43.0	-10.00	10.0	
		Week 8	Peg	19	15 (78.9)	14.53 (19.74)	-20.0	10.00	60.0	1.40 (0.52) [0.54, 2.58]
			SoC	10	8 (80.0)	-12.38 (15.69)	-34.0	-15.00	10.0	
		Week 12	Peg	19	17 (89.5)	8.24 (32.56)	-75.0	10.00	70.0	0.79 (0.47) [-0.05, 1.79]
			SoC	10	8 (80.0)	-14.88 (13.59)	-35.0	-20.00	3.0	
		Week 16	Peg	19	16 (84.2)	11.38 (32.43)	-75.0	10.00	60.0	0.65 (0.45) [-0.17, 1.60]
			SoC	10	9 (90.0)	-7.33 (16.62)	-36.0	-8.00	20.0	
		Week 20	Peg	19	17 (89.5)	17.65 (23.32)	-10.0	10.00	70.0	1.42 (0.50) [0.57, 2.56]
			SoC	10	8 (80.0)	-13.13 (13.86)	-40.0	-11.50	3.0	
		Week 26	Peg	19	14 (73.7)	18.79 (22.74)	-20.0	11.50	60.0	1.33 (0.57) [0.37, 2.63]
			SoC	10	6 (60.0)	-10.50 (15.85)	-38.0	-4.00	3.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SIHD: Change from baseline in LASA Daily Activities (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline in LASA Daily Activities (censored)	Female	Week 4	Peg	16	15 (93.8)	5.60 (18.63)	-20.0	0.00	49.0	-0.05 (0.48) [-1.01, 0.90]
			SoC	8	7 (87.5)	6.43 (12.87)	-7.0	0.00	30.0	
		Week 8	Peg	16	14 (87.5)	13.57 (21.31)	-20.0	8.00	59.0	0.37 (0.49) [-0.56, 1.40]
			SoC	8	7 (87.5)	6.14 (13.13)	-7.0	0.00	30.0	
		Week 12	Peg	16	14 (87.5)	12.86 (25.27)	-35.0	15.50	69.0	0.17 (0.49) [-0.78, 1.16]
			SoC	8	7 (87.5)	9.00 (13.11)	-7.0	5.00	30.0	
		Week 16	Peg	16	14 (87.5)	13.93 (25.09)	-20.0	8.50	69.0	0.15 (0.49) [-0.80, 1.14]
			SoC	8	7 (87.5)	10.43 (14.93)	-7.0	5.00	30.0	
		Week 20	Peg	16	15 (93.8)	15.07 (22.28)	-10.0	5.00	69.0	0.33 (0.49) [-0.60, 1.32]
			SoC	8	7 (87.5)	8.29 (13.50)	-7.0	0.00	30.0	
		Week 26	Peg	16	13 (81.3)	16.38 (22.24)	-20.0	10.00	69.0	0.32 (0.53) [-0.68, 1.42]
			SoC	8	6 (75.0)	9.50 (14.38)	-7.0	7.50	30.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SICB: Change from baseline in LASA Daily Activities (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.2211

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SICB: Change from baseline in LASA Daily Activities (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Latin America	Peg	Week 4	12	11 (91.7)	29.09 (3.95)	(20.15, 38.03)	35.80 (10.21)	(12.71, 58.89)	0.0066	*
		Week 8	12	10 (83.3)	29.47 (3.28)	(21.90, 37.03)	37.30 (8.21)	(18.37, 56.23)	0.0019	*
		Week 12	12	11 (91.7)	21.57 (6.18)	(7.60, 35.55)	35.01 (15.95)	(-1.08, 71.11)	0.0558	
		Week 16	12	10 (83.3)	24.45 (7.52)	(7.11, 41.79)	37.69 (18.82)	(-5.70, 81.08)	0.0802	
		Week 20	12	10 (83.3)	34.36 (3.28)	(26.81, 41.92)	41.68 (8.15)	(22.90, 60.46)	0.0009	*
		Week 26	12	9 (75.0)	33.78 (3.18)	(26.26, 41.29)	39.08 (7.51)	(21.33, 56.84)	0.0012	*
	SoC	Week 4	2	2 (100.0)	-6.71 (9.36)	(-27.87, 14.46)				
		Week 8	2	2 (100.0)	-7.84 (7.46)	(-25.04, 9.37)				
		Week 12	2	2 (100.0)	-13.44 (14.62)	(-46.52, 19.64)				
		Week 16	2	2 (100.0)	-13.24 (17.11)	(-52.69, 26.21)				
		Week 20	2	2 (100.0)	-7.32 (7.41)	(-24.41, 9.78)				
		Week 26	2	2 (100.0)	-5.31 (6.77)	(-21.31, 10.69)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SICB: Change from baseline in LASA Daily Activities (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Asia Pacific Peg	Peg	Week 4	23	20 (87.0)	2.51 (3.24)	(-4.13, 9.14)	7.98 (5.32)	(-2.91, 18.86)	0.1449	
		Week 8	23	19 (82.6)	8.40 (3.62)	(0.98, 15.83)	15.07 (5.87)	(3.05, 27.09)	0.0158	*
		Week 12	23	20 (87.0)	7.86 (3.79)	(0.11, 15.61)	15.66 (6.23)	(2.91, 28.40)	0.0178	*
		Week 16	23	20 (87.0)	9.24 (4.27)	(0.52, 17.96)	10.57 (6.77)	(-3.25, 24.40)	0.1287	
		Week 20	23	22 (95.7)	10.38 (3.47)	(3.31, 17.44)	16.81 (5.85)	(4.87, 28.75)	0.0073	*
	Week 26	23	18 (78.3)	11.41 (4.12)	(2.90, 19.93)	17.06 (7.35)	(1.89, 32.23)	0.0291	*	
	SoC	Week 4	16	13 (81.3)	-5.47 (4.09)	(-13.84, 2.90)				
		Week 8	16	13 (81.3)	-6.67 (4.43)	(-15.73, 2.40)				
		Week 12	16	13 (81.3)	-7.79 (4.79)	(-17.58, 2.00)				
		Week 16	16	14 (87.5)	-1.34 (5.13)	(-11.81, 9.14)				
Week 20		16	13 (81.3)	-6.44 (4.59)	(-15.80, 2.93)					
Week 26	16	10 (62.5)	-5.65 (5.92)	(-17.86, 6.57)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SIHB: Change from baseline in LASA Daily Activities (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Daily Activities (censored)	Latin America	Baseline	Peg	12	12 (100.0)	52.92 (26.06)	14.0	50.50	95.0	
			SoC	2	2 (100.0)	45.00 (7.07)	40.0	45.00	50.0	
		Week 4	Peg	12	11 (91.7)	84.09 (12.81)	60.0	90.00	100.0	
			SoC	2	2 (100.0)	46.50 (4.95)	43.0	46.50	50.0	
		Week 8	Peg	12	10 (83.3)	85.80 (10.32)	70.0	90.00	100.0	
			SoC	2	2 (100.0)	46.50 (4.95)	43.0	46.50	50.0	
		Week 12	Peg	12	11 (91.7)	74.45 (25.45)	10.0	80.00	100.0	
			SoC	2	2 (100.0)	46.50 (4.95)	43.0	46.50	50.0	
		Week 16	Peg	12	10 (83.3)	79.70 (25.14)	10.0	90.00	97.0	
			SoC	2	2 (100.0)	46.50 (4.95)	43.0	46.50	50.0	
		Week 20	Peg	12	10 (83.3)	88.80 (9.72)	70.0	90.00	100.0	
			SoC	2	2 (100.0)	46.50 (4.95)	43.0	46.50	50.0	
		Week 26	Peg	12	9 (75.0)	87.33 (9.90)	68.0	90.00	100.0	
			SoC	2	2 (100.0)	46.50 (4.95)	43.0	46.50	50.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SIHB: Change from baseline in LASA Daily Activities (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Daily Activities (censored)	Asia	Baseline	Peg	23	23 (100.0)	67.26 (20.32)	10.0	70.00	90.0	
			SoC	16	14 (87.5)	64.86 (15.10)	48.0	60.00	95.0	
	Pacific	Week 4	Peg	23	20 (87.0)	68.00 (21.59)	2.0	70.00	100.0	
			SoC	16	15 (93.8)	63.00 (21.05)	27.0	70.00	92.0	
	Week 8	Peg	23	19 (82.6)	73.63 (21.24)	20.0	80.00	100.0		
		SoC	16	14 (87.5)	60.21 (22.69)	20.0	67.50	90.0		
	Week 12	Peg	23	20 (87.0)	73.95 (23.69)	10.0	80.00	100.0		
		SoC	16	14 (87.5)	60.21 (20.96)	30.0	55.00	100.0		
	Week 16	Peg	23	20 (87.0)	74.30 (22.62)	20.0	80.00	100.0		
		SoC	16	15 (93.8)	65.73 (23.26)	30.0	65.00	100.0		
	Week 20	Peg	23	22 (95.7)	75.64 (21.11)	10.0	80.00	100.0		
		SoC	16	15 (93.8)	62.13 (20.65)	30.0	65.00	95.0		
	Week 26	Peg	23	18 (78.3)	76.94 (25.07)	10.0	83.50	100.0		
		SoC	16	11 (68.8)	62.55 (22.27)	30.0	65.00	94.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SIHB: Change from baseline in LASA Daily Activities (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline in LASA Daily Activities (censored)	Latin America	Week 4	Peg	12	11 (91.7)	27.64 (23.96)	-5.0	19.00	70.0	1.06	(0.89) [-0.47, 3.07]
			SoC	2	2 (100.0)	1.50 (2.12)	0.0	1.50	3.0		
		Week 8	Peg	12	10 (83.3)	27.60 (23.17)	0.0	22.00	60.0	1.10	(0.92) [-0.45, 3.19]
			SoC	2	2 (100.0)	1.50 (2.12)	0.0	1.50	3.0		
		Week 12	Peg	12	11 (91.7)	18.00 (43.06)	-75.0	21.00	70.0	0.37	(0.86) [-1.24, 2.16]
			SoC	2	2 (100.0)	1.50 (2.12)	0.0	1.50	3.0		
		Week 16	Peg	12	10 (83.3)	21.50 (40.97)	-75.0	25.00	69.0	0.47	(0.88) [-1.15, 2.34]
			SoC	2	2 (100.0)	1.50 (2.12)	0.0	1.50	3.0		
		Week 20	Peg	12	10 (83.3)	32.60 (26.01)	-5.0	34.50	70.0	1.16	(0.92) [-0.37, 3.28]
			SoC	2	2 (100.0)	1.50 (2.12)	0.0	1.50	3.0		
		Week 26	Peg	12	9 (75.0)	32.22 (23.76)	-5.0	29.00	69.0	1.25	(0.96) [-0.29, 3.51]
			SoC	2	2 (100.0)	1.50 (2.12)	0.0	1.50	3.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SIHB: Change from baseline in LASA Daily Activities (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline in LASA Daily Activities (censored)	Asia Pacific	Week 4	Peg	23	20 (87.0)	1.15 (13.56)	-20.0	0.00	30.0	0.26 (0.37)	[-0.45, 1.01]
			SoC	16	13 (81.3)	-2.92 (17.84)	-43.0	-3.00	30.0		
		Week 8	Peg	23	19 (82.6)	6.95 (14.42)	-20.0	7.00	30.0	0.70 (0.38)	[-0.01, 1.51]
			SoC	16	13 (81.3)	-4.54 (18.19)	-34.0	-5.00	30.0		
		Week 12	Peg	23	20 (87.0)	6.10 (17.60)	-35.0	6.50	40.0	0.57 (0.38)	[-0.13, 1.35]
			SoC	16	13 (81.3)	-4.54 (19.12)	-35.0	-2.00	30.0		
		Week 16	Peg	23	20 (87.0)	8.10 (20.14)	-30.0	6.00	50.0	0.39 (0.36)	[-0.30, 1.13]
			SoC	16	14 (87.5)	0.29 (19.18)	-36.0	0.00	30.0		
		Week 20	Peg	23	22 (95.7)	9.09 (16.64)	-10.0	2.00	50.0	0.73 (0.37)	[0.04, 1.51]
			SoC	16	13 (81.3)	-3.85 (18.47)	-40.0	-5.00	30.0		
		Week 26	Peg	23	18 (78.3)	10.33 (17.67)	-20.0	10.00	50.0	0.59 (0.42)	[-0.18, 1.47]
			SoC	16	10 (62.5)	-0.90 (19.65)	-38.0	-0.50	30.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SICA: Change from baseline in LASA Daily Activities (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.5537

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SICA: Change from baseline in LASA Daily Activities (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
< 4	Peg	Week 4	21	18 (85.7)	7.15 (4.68)	(-2.61, 16.91)	16.69 (10.03)	(-4.24, 37.62)	0.1118	
		Week 8	21	17 (81.0)	10.55 (4.38)	(1.37, 19.72)	20.81 (9.19)	(1.56, 40.05)	0.0355	*
		Week 12	21	18 (85.7)	5.12 (6.00)	(-7.40, 17.64)	23.75 (12.88)	(-3.12, 50.61)	0.0801	
		Week 16	21	17 (81.0)	5.89 (6.36)	(-7.38, 19.16)	9.74 (12.49)	(-16.31, 35.80)	0.4444	
		Week 20	21	18 (85.7)	14.22 (4.26)	(5.32, 23.11)	28.39 (9.15)	(9.30, 47.49)	0.0056	*
	Week 26	21	15 (71.4)	14.81 (5.63)	(2.80, 26.82)	28.86 (13.84)	(-0.64, 58.35)	0.0545		
	SoC	Week 4	8	5 (62.5)	-9.54 (8.88)	(-28.06, 8.97)				
		Week 8	8	5 (62.5)	-10.26 (8.08)	(-27.18, 6.66)				
		Week 12	8	5 (62.5)	-18.63 (11.39)	(-42.39, 5.14)				
		Week 16	8	6 (75.0)	-3.85 (10.73)	(-26.23, 18.52)				
		Week 20	8	5 (62.5)	-14.18 (8.10)	(-31.06, 2.71)				
Week 26	8	3 (37.5)	-14.05 (12.62)	(-40.95, 12.86)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SICA: Change from baseline in LASA Daily Activities (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	15.77 (4.46)	(6.45, 25.08)	15.16 (6.79)	(1.00, 29.32)	0.0371	*
		Week 8	14	12 (85.7)	19.56 (4.68)	(9.77, 29.34)	20.62 (6.95)	(6.08, 35.16)	0.0079	*
		Week 12	14	13 (92.9)	18.18 (4.54)	(8.71, 27.65)	15.31 (6.92)	(0.89, 29.74)	0.0386	*
		Week 16	14	13 (92.9)	20.90 (4.54)	(11.42, 30.38)	17.37 (6.92)	(2.92, 31.81)	0.0209	*
		Week 20	14	14 (100.0)	19.80 (4.56)	(10.32, 29.28)	18.12 (7.11)	(3.33, 32.90)	0.0187	*
		Week 26	14	12 (85.7)	22.32 (4.33)	(13.22, 31.43)	19.87 (6.64)	(5.92, 33.81)	0.0078	*
	SoC	Week 4	10	10 (100.0)	0.61 (5.09)	(-10.02, 11.23)				
		Week 8	10	10 (100.0)	-1.07 (5.12)	(-11.79, 9.66)				
		Week 12	10	10 (100.0)	2.87 (5.18)	(-7.94, 13.67)				
		Week 16	10	10 (100.0)	3.53 (5.19)	(-7.29, 14.35)				
		Week 20	10	10 (100.0)	1.68 (5.41)	(-9.56, 12.92)				
		Week 26	10	9 (90.0)	2.46 (5.01)	(-8.06, 12.98)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SIHA: Change from baseline in LASA Daily Activities (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Daily Activities (censored)	< 4	Baseline	Peg	21	21 (100.0)	61.00 (26.04)	10.0	70.00	95.0	
			SoC	8	6 (75.0)	67.50 (15.41)	40.0	72.50	80.0	
		Week 4	Peg	21	18 (85.7)	71.00 (23.41)	2.0	70.00	100.0	
			SoC	8	7 (87.5)	61.43 (21.66)	27.0	70.00	85.0	
		Week 8	Peg	21	17 (81.0)	75.71 (22.04)	20.0	80.00	100.0	
			SoC	8	6 (75.0)	58.00 (20.79)	36.0	56.50	85.0	
		Week 12	Peg	21	18 (85.7)	69.06 (27.88)	10.0	80.00	100.0	
			SoC	8	6 (75.0)	50.33 (14.49)	35.0	46.50	74.0	
		Week 16	Peg	21	17 (81.0)	70.00 (26.46)	10.0	80.00	90.0	
			SoC	8	7 (87.5)	63.00 (23.09)	34.0	60.00	100.0	
		Week 20	Peg	21	18 (85.7)	77.06 (20.62)	10.0	80.00	100.0	
			SoC	8	7 (87.5)	57.43 (18.55)	30.0	60.00	80.0	
		Week 26	Peg	21	15 (71.4)	76.33 (25.48)	10.0	87.00	100.0	
			SoC	8	4 (50.0)	52.25 (18.52)	32.0	51.50	74.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SIHA: Change from baseline in LASA Daily Activities (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Daily Activities (censored)	>= 4	Baseline	Peg	14	14 (100.0)	64.36 (18.64)	30.0	62.00	90.0	
			SoC	10	10 (100.0)	59.30 (15.86)	48.0	50.00	95.0	
		Week 4	Peg	14	13 (92.9)	77.46 (15.01)	50.0	80.00	100.0	
			SoC	10	10 (100.0)	60.80 (20.80)	30.0	57.50	92.0	
		Week 8	Peg	14	12 (85.7)	80.83 (13.78)	50.0	81.00	100.0	
			SoC	10	10 (100.0)	58.80 (23.28)	20.0	57.50	90.0	
		Week 12	Peg	14	13 (92.9)	81.15 (15.32)	51.0	80.00	100.0	
			SoC	10	10 (100.0)	63.40 (22.05)	30.0	57.50	100.0	
		Week 16	Peg	14	13 (92.9)	84.08 (15.67)	51.0	90.00	100.0	
			SoC	10	10 (100.0)	63.80 (23.69)	30.0	57.50	100.0	
		Week 20	Peg	14	14 (100.0)	83.21 (17.25)	50.0	90.00	100.0	
			SoC	10	10 (100.0)	62.30 (21.77)	30.0	57.50	95.0	
		Week 26	Peg	14	12 (85.7)	85.50 (14.96)	50.0	89.50	100.0	
			SoC	10	9 (90.0)	63.56 (22.46)	30.0	65.00	94.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SIHA: Change from baseline in LASA Daily Activities (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in LASA Daily Activities (censored)	Week 4	Peg	21	18 (85.7)	7.28 (22.69)	-20.0	0.00	51.0	0.75 (0.55) [-0.25, 1.91]
		SoC	8	5 (62.5)	-10.00 (20.36)	-43.0	-10.00	10.0	
	Week 8	Peg	21	17 (81.0)	10.53 (21.33)	-20.0	7.00	59.0	0.97 (0.56) [-0.03, 2.19]
		SoC	8	5 (62.5)	-10.20 (17.64)	-34.0	-10.00	10.0	
	Week 12	Peg	21	18 (85.7)	5.33 (33.76)	-75.0	6.00	69.0	0.77 (0.55) [-0.23, 1.94]
		SoC	8	5 (62.5)	-19.40 (13.94)	-35.0	-20.00	3.0	
	Week 16	Peg	21	17 (81.0)	6.76 (34.28)	-75.0	0.00	69.0	0.40 (0.50) [-0.55, 1.44]
		SoC	8	6 (75.0)	-6.33 (19.95)	-36.0	-3.50	20.0	
	Week 20	Peg	21	18 (85.7)	14.56 (24.37)	-10.0	0.00	69.0	1.25 (0.57) [0.26, 2.52]
		SoC	8	5 (62.5)	-15.40 (16.38)	-40.0	-15.00	3.0	
	Week 26	Peg	21	15 (71.4)	14.33 (25.66)	-20.0	10.00	69.0	0.98 (0.70) [-0.27, 2.52]
		SoC	8	3 (37.5)	-11.67 (22.85)	-38.0	0.00	3.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LDWC_SIHA: Change from baseline in LASA Daily Activities (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in LASA Daily Activities (censored)	Week 4	Peg	14	13 (92.9)	15.08 (20.41)	-10.0	10.00	70.0	0.73 (0.46) [-0.10, 1.72]
		SoC	10	10 (100.0)	1.50 (13.99)	-20.0	0.00	30.0	
	Week 8	Peg	14	12 (85.7)	19.08 (18.03)	0.0	14.00	60.0	1.08 (0.49) [0.24, 2.17]
		SoC	10	10 (100.0)	-0.50 (16.60)	-28.0	0.00	30.0	
	Week 12	Peg	14	13 (92.9)	17.23 (20.38)	-10.0	12.00	70.0	0.70 (0.46) [-0.12, 1.68]
		SoC	10	10 (100.0)	4.10 (14.28)	-20.0	0.00	30.0	
	Week 16	Peg	14	13 (92.9)	20.15 (18.01)	1.0	12.00	60.0	0.87 (0.47) [0.05, 1.89]
		SoC	10	10 (100.0)	4.50 (16.19)	-20.0	0.00	30.0	
	Week 20	Peg	14	14 (100.0)	18.86 (20.50)	-5.0	10.00	70.0	0.83 (0.46) [0.02, 1.82]
		SoC	10	10 (100.0)	3.00 (14.71)	-20.0	0.00	30.0	
	Week 26	Peg	14	12 (85.7)	21.75 (16.83)	0.0	21.00	60.0	1.09 (0.51) [0.23, 2.22]
		SoC	10	9 (90.0)	3.22 (15.59)	-20.0	0.00	30.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SICC: Change from baseline in LASA Overall QoL (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Age	Peg	Overall								0.2890

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SICC: Change from baseline in LASA Overall QoL (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
< 60	Peg	Week 4	29	26 (89.7)	9.99 (3.40)	(3.06, 16.92)	13.38 (6.35)	(0.46, 26.30)	0.0427	*
		Week 8	29	24 (82.8)	11.79 (3.43)	(4.80, 18.78)	18.33 (6.21)	(5.65, 31.00)	0.0060	*
		Week 12	29	26 (89.7)	14.92 (3.29)	(8.23, 21.62)	23.79 (6.13)	(11.32, 36.26)	0.0005	*
		Week 16	29	25 (86.2)	13.21 (3.50)	(6.08, 20.34)	15.37 (6.23)	(2.70, 28.04)	0.0189	*
		Week 20	29	27 (93.1)	15.23 (3.35)	(8.42, 22.04)	19.70 (6.32)	(6.84, 32.55)	0.0037	*
		Week 26	29	23 (79.3)	16.20 (3.76)	(8.50, 23.90)	19.96 (7.01)	(5.62, 34.31)	0.0080	*
	SoC	Week 4	13	11 (84.6)	-3.39 (5.27)	(-14.12, 7.33)				
		Week 8	13	11 (84.6)	-6.53 (5.09)	(-16.91, 3.84)				
		Week 12	13	11 (84.6)	-8.87 (5.09)	(-19.22, 1.49)				
		Week 16	13	12 (92.3)	-2.16 (5.08)	(-12.49, 8.17)				
		Week 20	13	11 (84.6)	-4.47 (5.30)	(-15.24, 6.30)				
		Week 26	13	10 (76.9)	-3.76 (5.82)	(-15.66, 8.14)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SICC: Change from baseline in LASA Overall QoL (censored) by age
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 60	Peg	Week 4	6	5 (83.3)	12.31 (6.72)	(-4.97, 29.59)	28.39 (11.04)	(0.01, 56.76)	0.0500	*
		Week 8	6	5 (83.3)	21.03 (2.78)	(13.90, 28.16)	22.93 (4.56)	(11.22, 34.65)	0.0040	*
		Week 12	6	5 (83.3)	-4.01 (11.88)	(-34.56, 26.54)	9.21 (19.52)	(-40.95, 59.38)	0.6567	
		Week 16	6	5 (83.3)	8.09 (13.09)	(-25.55, 41.73)	21.73 (21.15)	(-32.65, 76.11)	0.3514	
		Week 20	6	5 (83.3)	18.58 (3.96)	(8.40, 28.76)	20.53 (6.40)	(4.08, 36.99)	0.0238	*
		Week 26	6	4 (66.7)	20.55 (5.21)	(-1.87, 42.97)	32.54 (9.90)	(-10.05, 75.13)	0.0814	
	SoC	Week 4	5	4 (80.0)	-16.08 (7.72)	(-35.93, 3.77)				
		Week 8	5	4 (80.0)	-1.90 (3.19)	(-10.10, 6.29)				
		Week 12	5	4 (80.0)	-13.23 (13.65)	(-48.32, 21.86)				
		Week 16	5	4 (80.0)	-13.64 (15.20)	(-52.71, 25.43)				
		Week 20	5	4 (80.0)	-1.95 (4.60)	(-13.77, 9.87)				
		Week 26	5	2 (40.0)	-11.99 (8.27)	(-47.59, 23.61)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SIHC: Change from baseline in LASA Overall QoL (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Overall QoL (censored)	< 60	Baseline	Peg	29	29 (100.0)	63.41 (22.02)	20.0	60.00	100.0	
			SoC	13	12 (92.3)	72.33 (18.51)	45.0	80.00	95.0	
		Week 4	Peg	29	26 (89.7)	74.19 (19.51)	20.0	80.00	100.0	
			SoC	13	12 (92.3)	67.25 (22.66)	27.0	64.00	100.0	
		Week 8	Peg	29	24 (82.8)	76.33 (18.61)	30.0	80.00	100.0	
			SoC	13	11 (84.6)	63.00 (23.37)	20.0	63.00	90.0	
		Week 12	Peg	29	26 (89.7)	79.77 (19.37)	20.0	80.00	100.0	
			SoC	13	11 (84.6)	60.73 (19.18)	35.0	60.00	90.0	
		Week 16	Peg	29	25 (86.2)	79.04 (19.12)	30.0	90.00	100.0	
			SoC	13	12 (92.3)	68.42 (23.18)	33.0	64.00	100.0	
		Week 20	Peg	29	27 (93.1)	80.11 (19.12)	10.0	85.00	100.0	
			SoC	13	12 (92.3)	66.33 (21.53)	30.0	64.00	98.0	
		Week 26	Peg	29	23 (79.3)	79.26 (20.39)	20.0	80.00	100.0	
			SoC	13	10 (76.9)	64.50 (21.13)	33.0	61.50	94.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SIHC: Change from baseline in LASA Overall QoL (censored) by age
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Overall QoL (censored)	>= 60	Baseline	Peg	6	6 (100.0)	69.17 (13.04)	50.0	73.50	85.0	
			SoC	5	4 (80.0)	50.00 (28.28)	10.0	60.00	70.0	
		Week 4	Peg	6	5 (83.3)	75.20 (14.92)	60.0	71.00	100.0	
			SoC	5	5 (100.0)	51.00 (15.97)	30.0	50.00	75.0	
		Week 8	Peg	6	5 (83.3)	86.60 (13.30)	66.0	90.00	100.0	
			SoC	5	5 (100.0)	58.00 (10.95)	50.0	50.00	70.0	
		Week 12	Peg	6	5 (83.3)	57.20 (27.73)	10.0	70.00	80.0	
			SoC	5	5 (100.0)	56.00 (8.94)	50.0	50.00	70.0	
		Week 16	Peg	6	5 (83.3)	65.40 (32.80)	10.0	70.00	90.0	
			SoC	5	5 (100.0)	54.00 (8.94)	50.0	50.00	70.0	
		Week 20	Peg	6	5 (83.3)	81.20 (17.81)	56.0	90.00	100.0	
			SoC	5	5 (100.0)	56.00 (8.94)	50.0	50.00	70.0	
		Week 26	Peg	6	4 (66.7)	88.00 (11.66)	72.0	90.00	100.0	
			SoC	5	3 (60.0)	56.67 (11.55)	50.0	50.00	70.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SIHC: Change from baseline in LASA Overall QoL (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 60 in LASA Overall QoL (censored)	Week 4	Peg	29	26 (89.7)	10.38 (18.74)	-20.0	9.50	51.0	0.77 (0.38) [0.07, 1.58]
		SoC	13	11 (84.6)	-4.64 (19.68)	-53.0	0.00	15.0	
	Week 8	Peg	29	24 (82.8)	12.21 (18.76)	-10.0	4.50	51.0	1.05 (0.40) [0.33, 1.90]
		SoC	13	11 (84.6)	-7.73 (18.32)	-45.0	0.00	15.0	
	Week 12	Peg	29	26 (89.7)	15.19 (20.44)	-20.0	10.50	61.0	1.20 (0.40) [0.48, 2.06]
		SoC	13	11 (84.6)	-10.00 (21.07)	-45.0	0.00	15.0	
	Week 16	Peg	29	25 (86.2)	13.88 (20.77)	-10.0	5.00	61.0	0.87 (0.38) [0.18, 1.67]
		SoC	13	12 (92.3)	-3.92 (18.57)	-47.0	2.50	15.0	
	Week 20	Peg	29	27 (93.1)	15.70 (21.30)	-20.0	10.00	61.0	1.00 (0.39) [0.30, 1.83]
		SoC	13	11 (84.6)	-5.64 (19.45)	-50.0	0.00	15.0	
	Week 26	Peg	29	23 (79.3)	16.48 (22.38)	-20.0	10.00	61.0	0.92 (0.41) [0.18, 1.80]
		SoC	13	10 (76.9)	-3.80 (19.22)	-47.0	2.00	15.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SIHC: Change from baseline in LASA Overall QoL (censored) by age
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 60 in LASA Overall QoL (censored)	Week 4	Peg	6	5 (83.3)	2.20 (7.92)	-6.0	1.00	15.0	0.28 (0.80) [-1.21, 1.98]
		SoC	5	4 (80.0)	-5.00 (34.16)	-40.0	-10.00	40.0	
	Week 8	Peg	6	5 (83.3)	13.60 (4.88)	8.0	15.00	19.0	0.45 (0.81) [-0.99, 2.24]
		SoC	5	4 (80.0)	5.00 (25.17)	-20.0	0.00	40.0	
	Week 12	Peg	6	5 (83.3)	-15.80 (34.88)	-75.0	-4.00	13.0	-0.52 (0.82) [-2.34, 0.91]
		SoC	5	4 (80.0)	2.50 (26.30)	-20.0	-5.00	40.0	
	Week 16	Peg	6	5 (83.3)	-3.00 (43.28)	-75.0	10.00	40.0	-0.07 (0.79) [-1.69, 1.49]
		SoC	5	4 (80.0)	0.00 (28.28)	-20.0	-10.00	40.0	
	Week 20	Peg	6	5 (83.3)	12.80 (17.46)	-4.0	14.00	40.0	0.42 (0.81) [-1.02, 2.20]
		SoC	5	4 (80.0)	2.50 (26.30)	-20.0	-5.00	40.0	
	Week 26	Peg	6	4 (66.7)	15.00 (14.31)	-2.0	14.50	33.0	1.40 (1.54) [-0.33, 5.38]
		SoC	5	2 (40.0)	-10.00 (14.14)	-20.0	-10.00	0.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SICD: Change from baseline in LASA Overall QoL (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA				
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA	
Sex	Peg	Overall									0.3132

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SICD: Change from baseline in LASA Overall QoL (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Male	Peg	Week 4	19	16 (84.2)	8.85 (4.44)	(-0.41, 18.11)	23.77 (7.67)	(7.77, 39.77)	0.0057	*
		Week 8	19	15 (78.9)	9.32 (4.13)	(0.67, 17.97)	22.67 (7.04)	(7.93, 37.40)	0.0045	*
		Week 12	19	17 (89.5)	5.68 (5.00)	(-4.72, 16.09)	22.27 (8.89)	(3.79, 40.76)	0.0205	*
		Week 16	19	16 (84.2)	6.35 (5.79)	(-5.70, 18.39)	17.08 (9.78)	(-3.26, 37.41)	0.0954	
		Week 20	19	17 (89.5)	13.31 (3.67)	(5.67, 20.94)	27.05 (6.51)	(13.52, 40.58)	0.0004	*
		Week 26	19	14 (73.7)	14.47 (4.31)	(5.32, 23.62)	28.40 (8.01)	(11.42, 45.39)	0.0027	*
	SoC	Week 4	10	8 (80.0)	-14.92 (6.31)	(-28.09, -1.76)				
		Week 8	10	8 (80.0)	-13.35 (5.72)	(-25.31, -1.38)				
		Week 12	10	8 (80.0)	-16.59 (7.36)	(-31.90, -1.29)				
		Week 16	10	9 (90.0)	-10.73 (7.88)	(-27.12, 5.66)				
		Week 20	10	8 (80.0)	-13.74 (5.40)	(-24.96, -2.52)				
		Week 26	10	6 (60.0)	-13.93 (6.66)	(-28.04, 0.18)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SICD: Change from baseline in LASA Overall QoL (censored) by sex
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Female	Peg	Week 4	16	15 (93.8)	10.01 (4.13)	(1.33, 18.69)	7.75 (8.74)	(-10.60, 26.11)	0.3867	
		Week 8	16	14 (87.5)	17.00 (3.85)	(8.88, 25.12)	17.07 (8.10)	(-0.03, 34.16)	0.0503	
		Week 12	16	14 (87.5)	17.53 (4.44)	(8.16, 26.91)	14.61 (9.35)	(-5.13, 34.34)	0.1368	
		Week 16	16	14 (87.5)	18.62 (4.34)	(9.46, 27.78)	19.30 (9.14)	(0.02, 38.58)	0.0498	*
		Week 20	16	15 (93.8)	18.82 (4.57)	(9.21, 28.43)	18.98 (9.67)	(-1.34, 39.29)	0.0653	
		Week 26	16	13 (81.3)	19.05 (5.37)	(7.62, 30.49)	19.66 (11.66)	(-5.20, 44.53)	0.1125	
	SoC	Week 4	8	7 (87.5)	2.26 (7.23)	(-12.93, 17.45)				
		Week 8	8	7 (87.5)	-0.07 (6.49)	(-13.77, 13.63)				
		Week 12	8	7 (87.5)	2.93 (7.50)	(-12.89, 18.75)				
		Week 16	8	7 (87.5)	-0.68 (7.33)	(-16.13, 14.78)				
		Week 20	8	7 (87.5)	-0.16 (8.00)	(-16.97, 16.66)				
		Week 26	8	6 (75.0)	-0.61 (9.48)	(-20.82, 19.60)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SIHD: Change from baseline in LASA Overall QoL (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Overall QoL (censored)	Male	Baseline	Peg	19	19 (100.0)	67.84 (18.38)	40.0	70.00	100.0	
			SoC	10	9 (90.0)	67.56 (18.30)	45.0	70.00	95.0	
		Week 4	Peg	19	16 (84.2)	76.00 (17.51)	50.0	75.00	100.0	
			SoC	10	9 (90.0)	53.78 (21.85)	27.0	50.00	95.0	
		Week 8	Peg	19	15 (78.9)	77.67 (18.48)	40.0	82.00	100.0	
			SoC	10	9 (90.0)	55.00 (20.62)	20.0	50.00	90.0	
		Week 12	Peg	19	17 (89.5)	72.94 (24.76)	10.0	70.00	100.0	
			SoC	10	9 (90.0)	51.11 (10.83)	35.0	50.00	70.0	
		Week 16	Peg	19	16 (84.2)	74.63 (24.35)	10.0	80.00	100.0	
			SoC	10	10 (100.0)	58.30 (20.00)	33.0	50.00	100.0	
		Week 20	Peg	19	17 (89.5)	79.88 (16.75)	50.0	80.00	100.0	
			SoC	10	9 (90.0)	54.44 (15.09)	30.0	50.00	80.0	
		Week 26	Peg	19	14 (73.7)	80.00 (18.40)	50.0	85.00	100.0	
			SoC	10	7 (70.0)	51.86 (12.68)	33.0	50.00	70.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SIHD: Change from baseline in LASA Overall QoL (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Overall QoL (censored)	Female	Baseline	Peg	16	16 (100.0)	60.31 (23.12)	20.0	55.00	100.0	
			SoC	8	7 (87.5)	65.71 (28.78)	10.0	80.00	90.0	
		Week 4	Peg	16	15 (93.8)	72.60 (20.21)	20.0	80.00	100.0	
			SoC	8	8 (100.0)	72.25 (18.26)	50.0	72.50	100.0	
		Week 8	Peg	16	14 (87.5)	78.57 (18.24)	30.0	80.00	100.0	
			SoC	8	7 (87.5)	69.71 (17.19)	50.0	65.00	90.0	
		Week 12	Peg	16	14 (87.5)	80.00 (18.40)	20.0	80.00	100.0	
			SoC	8	7 (87.5)	69.71 (17.19)	50.0	65.00	90.0	
		Week 16	Peg	16	14 (87.5)	79.21 (19.21)	30.0	90.00	96.0	
			SoC	8	7 (87.5)	72.57 (20.53)	50.0	65.00	100.0	
		Week 20	Peg	16	15 (93.8)	80.73 (21.19)	10.0	90.00	97.0	
			SoC	8	8 (100.0)	73.25 (18.80)	50.0	72.50	98.0	
		Week 26	Peg	16	13 (81.3)	81.15 (21.23)	20.0	90.00	100.0	
			SoC	8	6 (75.0)	75.33 (18.33)	50.0	77.50	94.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SIHD: Change from baseline in LASA Overall QoL (censored) by sex
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Male in LASA Overall QoL (censored)	Week 4	Peg	19	16 (84.2)	8.56 (17.64)	-10.0	2.00	50.0	1.10 (0.49) [0.25, 2.18]
		SoC	10	8 (80.0)	-13.63 (22.90)	-53.0	-5.50	15.0	
	Week 8	Peg	19	15 (78.9)	9.07 (15.88)	-10.0	8.00	50.0	1.18 (0.50) [0.32, 2.30]
		SoC	10	8 (80.0)	-11.63 (18.81)	-45.0	-7.50	15.0	
	Week 12	Peg	19	17 (89.5)	4.18 (27.31)	-75.0	5.00	50.0	0.76 (0.47) [-0.08, 1.76]
		SoC	10	8 (80.0)	-16.00 (21.17)	-45.0	-15.00	15.0	
	Week 16	Peg	19	16 (84.2)	6.13 (28.33)	-75.0	2.50	50.0	0.64 (0.45) [-0.18, 1.58]
		SoC	10	9 (90.0)	-10.56 (18.44)	-47.0	-10.00	15.0	
	Week 20	Peg	19	17 (89.5)	13.06 (18.90)	-10.0	7.00	50.0	1.30 (0.50) [0.46, 2.41]
		SoC	10	8 (80.0)	-12.25 (18.57)	-50.0	-10.00	15.0	
	Week 26	Peg	19	14 (73.7)	12.79 (18.71)	-11.0	9.00	50.0	1.12 (0.56) [0.16, 2.36]
		SoC	10	6 (60.0)	-10.00 (21.44)	-47.0	-4.00	15.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.

N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.

95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.

Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.

Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SIHD: Change from baseline in LASA Overall QoL (censored) by sex
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline in LASA Overall QoL (censored)	Female	Week 4	Peg	16	15 (93.8)	9.60 (18.20)	-20.0	9.00	51.0	0.21	(0.48) [-0.72, 1.20]
			SoC	8	7 (87.5)	5.43 (19.78)	-27.0	5.00	40.0		
		Week 8	Peg	16	14 (87.5)	16.07 (18.21)	-10.0	14.50	51.0	0.62	(0.50) [-0.30, 1.69]
			SoC	8	7 (87.5)	4.00 (19.76)	-27.0	0.00	40.0		
		Week 12	Peg	16	14 (87.5)	17.50 (21.57)	-20.0	20.00	61.0	0.62	(0.50) [-0.30, 1.68]
			SoC	8	7 (87.5)	4.00 (19.76)	-27.0	0.00	40.0		
		Week 16	Peg	16	14 (87.5)	16.71 (21.75)	-10.0	12.00	61.0	0.45	(0.50) [-0.48, 1.48]
			SoC	8	7 (87.5)	6.86 (19.68)	-27.0	10.00	40.0		
		Week 20	Peg	16	15 (93.8)	17.73 (22.61)	-20.0	15.00	61.0	0.49	(0.49) [-0.42, 1.52]
			SoC	8	7 (87.5)	6.57 (19.65)	-27.0	8.00	40.0		
		Week 26	Peg	16	13 (81.3)	20.00 (23.70)	-20.0	20.00	61.0	0.88	(0.55) [-0.09, 2.10]
			SoC	8	6 (75.0)	0.33 (13.92)	-27.0	4.50	10.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SICB: Change from baseline in LASA Overall QoL (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Region	Peg	Overall								0.5694

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SICB: Change from baseline in LASA Overall QoL (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Latin America	Peg	Week 4	12	11 (91.7)	23.06 (3.58)	(14.96, 31.15)	27.14 (9.44)	(5.78, 48.50)	0.0184	*
		Week 8	12	10 (83.3)	24.85 (3.50)	(16.78, 32.91)	29.59 (8.95)	(8.95, 50.22)	0.0108	*
		Week 12	12	11 (91.7)	20.03 (6.66)	(4.96, 35.09)	31.92 (17.58)	(-7.84, 71.68)	0.1027	
		Week 16	12	10 (83.3)	19.20 (7.65)	(1.57, 36.84)	31.71 (19.57)	(-13.41, 76.83)	0.1438	
		Week 20	12	10 (83.3)	30.15 (3.64)	(21.75, 38.54)	32.37 (9.21)	(11.14, 53.60)	0.0079	*
		Week 26	12	9 (75.0)	29.77 (4.37)	(19.44, 40.10)	29.22 (10.46)	(4.49, 53.96)	0.0268	*
	SoC	Week 4	2	2 (100.0)	-4.08 (8.61)	(-23.56, 15.40)				
		Week 8	2	2 (100.0)	-4.74 (8.10)	(-23.42, 13.94)				
		Week 12	2	2 (100.0)	-11.90 (16.03)	(-48.16, 24.37)				
		Week 16	2	2 (100.0)	-12.51 (17.71)	(-53.35, 28.34)				
		Week 20	2	2 (100.0)	-2.23 (8.35)	(-21.49, 17.03)				
		Week 26	2	2 (100.0)	0.55 (9.38)	(-21.64, 22.74)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SICB: Change from baseline in LASA Overall QoL (censored) by region
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			IA
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	
Asia Pacific Peg		Week 4	23	20 (87.0)	3.20 (3.66)	(-4.29, 10.69)	11.11 (6.02)	(-1.19, 23.41)	0.0750	
		Week 8	23	19 (82.6)	7.78 (3.50)	(0.61, 14.95)	14.77 (5.66)	(3.17, 26.37)	0.0145	*
		Week 12	23	20 (87.0)	8.40 (3.53)	(1.18, 15.61)	19.73 (5.79)	(7.89, 31.58)	0.0019	*
		Week 16	23	20 (87.0)	9.43 (3.82)	(1.63, 17.23)	14.48 (6.06)	(2.10, 26.86)	0.0234	*
		Week 20	23	22 (95.7)	9.98 (3.65)	(2.54, 17.42)	16.50 (6.15)	(3.95, 29.05)	0.0116	*
		Week 26	23	18 (78.3)	10.34 (4.19)	(1.68, 18.99)	20.12 (7.43)	(4.77, 35.46)	0.0123	*
SoC		Week 4	16	13 (81.3)	-7.91 (4.63)	(-17.37, 1.55)				
		Week 8	16	13 (81.3)	-6.99 (4.27)	(-15.74, 1.76)				
		Week 12	16	13 (81.3)	-11.34 (4.46)	(-20.45, -2.22)				
		Week 16	16	14 (87.5)	-5.05 (4.59)	(-14.42, 4.33)				
		Week 20	16	13 (81.3)	-6.52 (4.83)	(-16.37, 3.33)				
		Week 26	16	10 (62.5)	-9.78 (6.00)	(-22.16, 2.60)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, number PRBC transfusions previous year (<4, >=4), covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SIHB: Change from baseline in LASA Overall QoL (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Overall QoL (censored)	Latin America	Baseline	Peg	12	12 (100.0)	58.75 (22.66)	20.0	55.00	90.0	
			SoC	2	2 (100.0)	47.50 (3.54)	45.0	47.50	50.0	
		Week 4	Peg	12	11 (91.7)	83.18 (11.02)	70.0	80.00	100.0	
			SoC	2	2 (100.0)	55.00 (7.07)	50.0	55.00	60.0	
		Week 8	Peg	12	10 (83.3)	85.90 (10.40)	70.0	90.00	100.0	
			SoC	2	2 (100.0)	55.00 (7.07)	50.0	55.00	60.0	
		Week 12	Peg	12	11 (91.7)	78.09 (24.33)	10.0	80.00	100.0	
			SoC	2	2 (100.0)	55.00 (7.07)	50.0	55.00	60.0	
		Week 16	Peg	12	10 (83.3)	78.70 (25.32)	10.0	90.00	97.0	
			SoC	2	2 (100.0)	55.00 (7.07)	50.0	55.00	60.0	
		Week 20	Peg	12	10 (83.3)	88.70 (10.71)	70.0	90.00	100.0	
			SoC	2	2 (100.0)	55.00 (7.07)	50.0	55.00	60.0	
		Week 26	Peg	12	9 (75.0)	87.11 (12.97)	56.0	90.00	100.0	
			SoC	2	2 (100.0)	55.00 (7.07)	50.0	55.00	60.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SIHB: Change from baseline in LASA Overall QoL (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Overall QoL (censored)	Asia Pacific	Baseline	Peg	23	23 (100.0)	67.35 (19.49)	30.0	70.00	100.0	
			SoC	16	14 (87.5)	69.50 (22.88)	10.0	75.00	95.0	
		Week 4	Peg	23	20 (87.0)	69.50 (20.35)	20.0	70.50	100.0	
	SoC		16	15 (93.8)	63.47 (23.02)	27.0	63.00	100.0		
		Week 8	Peg	23	19 (82.6)	74.00 (20.02)	30.0	80.00	100.0	
	SoC		16	14 (87.5)	62.36 (21.27)	20.0	64.00	90.0		
		Week 12	Peg	23	20 (87.0)	75.05 (21.28)	20.0	80.00	100.0	
	SoC		16	14 (87.5)	59.86 (17.51)	35.0	55.00	90.0		
		Week 16	Peg	23	20 (87.0)	75.80 (20.55)	30.0	80.00	100.0	
	SoC		16	15 (93.8)	65.40 (21.92)	33.0	63.00	100.0		
		Week 20	Peg	23	22 (95.7)	76.45 (20.38)	10.0	80.00	100.0	
	SoC		16	15 (93.8)	64.40 (19.98)	30.0	63.00	98.0		
		Week 26	Peg	23	18 (78.3)	77.28 (21.55)	20.0	80.00	100.0	
	SoC		16	11 (68.8)	64.09 (20.58)	33.0	63.00	94.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SIHB: Change from baseline in LASA Overall QoL (censored) by region
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]	
Change from baseline in LASA Overall QoL (censored)	Latin America	Week 4	Peg	12	11 (91.7)	20.91 (21.34)	-10.0	20.00	51.0	0.61	(0.86) [-0.97, 2.46]
			SoC	2	2 (100.0)	7.50 (10.61)	0.0	7.50	15.0		
		Week 8	Peg	12	10 (83.3)	22.40 (21.63)	-10.0	24.50	51.0	0.66	(0.89) [-0.93, 2.59]
			SoC	2	2 (100.0)	7.50 (10.61)	0.0	7.50	15.0		
		Week 12	Peg	12	11 (91.7)	15.82 (37.20)	-75.0	24.00	61.0	0.22	(0.85) [-1.43, 1.96]
			SoC	2	2 (100.0)	7.50 (10.61)	0.0	7.50	15.0		
		Week 16	Peg	12	10 (83.3)	15.20 (38.31)	-75.0	22.00	61.0	0.19	(0.87) [-1.48, 1.97]
			SoC	2	2 (100.0)	7.50 (10.61)	0.0	7.50	15.0		
		Week 20	Peg	12	10 (83.3)	28.20 (20.99)	-5.0	30.00	61.0	0.95	(0.90) [-0.61, 2.98]
			SoC	2	2 (100.0)	7.50 (10.61)	0.0	7.50	15.0		
		Week 26	Peg	12	9 (75.0)	28.22 (19.28)	6.0	30.00	61.0	1.02	(0.94) [-0.54, 3.17]
			SoC	2	2 (100.0)	7.50 (10.61)	0.0	7.50	15.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SIHB: Change from baseline in LASA Overall QoL (censored) by region
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline Asia in LASA Overall QoL Pacific (censored)	Week 4	Peg	23	20 (87.0)	2.55 (11.16)	-20.0	0.00	30.0	0.52 (0.37) [-0.18, 1.29]
		SoC	16	13 (81.3)	-6.62 (24.01)	-53.0	0.00	40.0	
	Week 8	Peg	23	19 (82.6)	7.21 (11.73)	-10.0	5.00	30.0	0.81 (0.39) [0.10, 1.64]
		SoC	16	13 (81.3)	-6.15 (21.01)	-45.0	0.00	40.0	
	Week 12	Peg	23	20 (87.0)	7.10 (16.17)	-20.0	9.50	30.0	0.81 (0.38) [0.11, 1.63]
		SoC	16	13 (81.3)	-8.85 (23.09)	-45.0	-3.00	40.0	
	Week 16	Peg	23	20 (87.0)	9.00 (17.12)	-10.0	0.00	40.0	0.69 (0.37) [0.01, 1.47]
		SoC	16	14 (87.5)	-4.43 (21.26)	-47.0	-4.00	40.0	
	Week 20	Peg	23	22 (95.7)	9.36 (17.79)	-20.0	0.00	40.0	0.73 (0.37) [0.05, 1.52]
		SoC	16	13 (81.3)	-5.15 (21.72)	-50.0	-8.00	40.0	
	Week 26	Peg	23	18 (78.3)	10.28 (19.91)	-20.0	5.00	45.0	0.88 (0.43) [0.10, 1.80]
		SoC	16	10 (62.5)	-7.30 (18.59)	-47.0	0.00	10.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SICA: Change from baseline in LASA Overall QoL (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	ANCOVA					
					Change from Baseline		Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
Number of transfusions in the previous year	Peg	Overall								0.7353

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SICA: Change from baseline in LASA Overall QoL (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
< 4	Peg	Week 4	21	18 (85.7)	7.39 (4.37)	(-1.73, 16.51)	16.19 (9.45)	(-3.53, 35.91)	0.1023	
		Week 8	21	17 (81.0)	11.53 (4.07)	(3.02, 20.05)	18.75 (8.59)	(0.78, 36.71)	0.0417	*
		Week 12	21	18 (85.7)	6.50 (5.74)	(-5.48, 18.48)	23.29 (12.41)	(-2.61, 49.18)	0.0753	
		Week 16	21	17 (81.0)	6.67 (6.01)	(-5.87, 19.22)	12.08 (12.01)	(-12.97, 37.13)	0.3265	
		Week 20	21	18 (85.7)	14.71 (4.35)	(5.65, 23.78)	24.68 (9.44)	(4.98, 44.37)	0.0166	*
	Week 26	21	15 (71.4)	15.18 (5.38)	(3.70, 26.65)	25.06 (13.19)	(-3.05, 53.17)	0.0768		
	SoC	Week 4	8	5 (62.5)	-8.80 (8.34)	(-26.21, 8.61)				
		Week 8	8	5 (62.5)	-7.21 (7.53)	(-22.98, 8.55)				
		Week 12	8	5 (62.5)	-16.79 (10.96)	(-39.65, 6.07)				
		Week 16	8	6 (75.0)	-5.41 (10.25)	(-26.80, 15.98)				
		Week 20	8	5 (62.5)	-9.97 (8.32)	(-27.33, 7.40)				
Week 26		8	3 (37.5)	-9.88 (12.04)	(-35.54, 15.77)					

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SICA: Change from baseline in LASA Overall QoL (censored) by number of transfusions previous year
 ITT

Subgroup	Treatment	Time point	N	n (%)	Change from Baseline		ANCOVA Treatment Difference			
					LS-Mean (SE)	95% CI	LS-Mean (SE)	95% CI	p-value	IA
>= 4	Peg	Week 4	14	13 (92.9)	11.32 (4.48)	(1.98, 20.66)	13.93 (6.82)	(-0.30, 28.16)	0.0545	
		Week 8	14	12 (85.7)	14.29 (4.36)	(5.16, 23.42)	17.83 (6.50)	(4.23, 31.43)	0.0129	*
		Week 12	14	13 (92.9)	15.59 (3.48)	(8.32, 22.85)	17.55 (5.32)	(6.44, 28.65)	0.0036	*
		Week 16	14	13 (92.9)	15.75 (4.24)	(6.90, 24.60)	15.82 (6.48)	(2.31, 29.34)	0.0240	*
		Week 20	14	14 (100.0)	16.08 (4.31)	(7.12, 25.04)	16.49 (6.73)	(2.50, 30.49)	0.0231	*
	Week 26	14	12 (85.7)	17.53 (4.08)	(8.95, 26.11)	20.56 (6.24)	(7.46, 33.67)	0.0040	*	
	SoC	Week 4	10	10 (100.0)	-2.61 (5.11)	(-13.27, 8.05)				
		Week 8	10	10 (100.0)	-3.54 (4.78)	(-13.55, 6.47)				
		Week 12	10	10 (100.0)	-1.96 (3.98)	(-10.26, 6.34)				
		Week 16	10	10 (100.0)	-0.07 (4.84)	(-10.18, 10.03)				
		Week 20	10	10 (100.0)	-0.41 (5.11)	(-11.04, 10.22)				
Week 26	10	9 (90.0)	-3.04 (4.72)	(-12.94, 6.87)						

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. n(%) = number of non-missing values included in the model. LS-Mean = Least square mean.
 ANCOVA = Analysis of covariance. SE = Standard error. 95% CI = 95% confidence interval. * = significant treatment effect.
 ANCOVA: fixed effects = treatment, covariate = baseline.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg. NE = not evaluable. IA = p-value of interaction term.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SIHA: Change from baseline in LASA Overall QoL (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Overall QoL (censored)	< 4	Baseline	Peg	21	21 (100.0)	60.86 (21.59)	20.0	70.00	90.0	
			SoC	8	6 (75.0)	73.33 (18.89)	45.0	75.00	95.0	
		Week 4	Peg	21	18 (85.7)	71.39 (19.42)	20.0	70.50	100.0	
			SoC	8	7 (87.5)	63.86 (22.12)	27.0	60.00	95.0	
		Week 8	Peg	21	17 (81.0)	76.06 (20.70)	30.0	80.00	100.0	
			SoC	8	6 (75.0)	62.50 (18.91)	35.0	65.00	90.0	
		Week 12	Peg	21	18 (85.7)	70.89 (26.20)	10.0	80.00	100.0	
			SoC	8	6 (75.0)	52.50 (13.32)	35.0	55.00	70.0	
		Week 16	Peg	21	17 (81.0)	71.35 (24.85)	10.0	80.00	95.0	
			SoC	8	7 (87.5)	63.29 (22.19)	33.0	60.00	100.0	
		Week 20	Peg	21	18 (85.7)	77.22 (20.02)	10.0	80.00	100.0	
			SoC	8	7 (87.5)	61.43 (17.73)	30.0	60.00	80.0	
		Week 26	Peg	21	15 (71.4)	75.67 (21.80)	20.0	80.00	100.0	
			SoC	8	4 (50.0)	55.75 (15.88)	33.0	60.00	70.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SIHA: Change from baseline in LASA Overall QoL (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup	Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
LASA Overall QoL (censored)	>= 4	Baseline	Peg	14	14 (100.0)	69.71 (18.83)	50.0	65.00	100.0	
			SoC	10	10 (100.0)	62.80 (24.64)	10.0	65.00	90.0	
	Week 4	Peg	14	13 (92.9)	78.46 (17.37)	50.0	80.00	100.0		
		SoC	10	10 (100.0)	61.50 (22.71)	30.0	56.50	100.0		
	Week 8	Peg	14	12 (85.7)	81.00 (13.76)	50.0	81.00	100.0		
		SoC	10	10 (100.0)	60.80 (21.67)	20.0	56.50	90.0		
	Week 12	Peg	14	13 (92.9)	83.38 (12.03)	70.0	80.00	100.0		
		SoC	10	10 (100.0)	63.30 (17.48)	45.0	56.50	90.0		
	Week 16	Peg	14	13 (92.9)	83.85 (15.33)	50.0	90.00	100.0		
		SoC	10	10 (100.0)	64.80 (21.09)	40.0	56.50	100.0		
	Week 20	Peg	14	14 (100.0)	84.21 (16.61)	50.0	90.00	100.0		
		SoC	10	10 (100.0)	64.60 (20.73)	40.0	56.50	98.0		
	Week 26	Peg	14	12 (85.7)	86.67 (14.63)	50.0	90.00	100.0		
		SoC	10	9 (90.0)	65.78 (20.58)	40.0	63.00	94.0		

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SIHA: Change from baseline in LASA Overall QoL (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline < 4 in LASA Overall QoL (censored)	Week 4	Peg	21	18 (85.7)	8.17 (19.62)	-20.0	4.50	51.0	0.90 (0.55) [-0.09, 2.10]
		SoC	8	5 (62.5)	-11.60 (26.27)	-53.0	0.00	15.0	
	Week 8	Peg	21	17 (81.0)	12.06 (17.02)	-10.0	8.00	51.0	1.11 (0.57) [0.12, 2.37]
		SoC	8	5 (62.5)	-9.00 (22.19)	-45.0	-5.00	15.0	
	Week 12	Peg	21	18 (85.7)	7.67 (29.50)	-75.0	7.00	61.0	0.96 (0.56) [-0.03, 2.16]
		SoC	8	5 (62.5)	-21.00 (25.35)	-45.0	-20.00	15.0	
	Week 16	Peg	21	17 (81.0)	8.71 (30.01)	-75.0	0.00	61.0	0.67 (0.51) [-0.26, 1.76]
		SoC	8	6 (75.0)	-11.17 (22.41)	-47.0	-12.50	15.0	
	Week 20	Peg	21	18 (85.7)	15.83 (21.22)	-20.0	15.00	61.0	1.33 (0.58) [0.34, 2.61]
		SoC	8	5 (62.5)	-14.00 (23.29)	-50.0	-10.00	15.0	
	Week 26	Peg	21	15 (71.4)	15.33 (23.90)	-20.0	10.00	61.0	0.99 (0.70) [-0.26, 2.53]
		SoC	8	3 (37.5)	-10.67 (32.35)	-47.0	0.00	15.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LQWC_SIHA: Change from baseline in LASA Overall QoL (censored) by number of transfusions previous year
 ITT

Parameter	Subgroup Time	Treatment	N	n (%)	Mean (SD)	Min	Q50	Max	Hedge's g (SE) [95% CI]
Change from baseline >= 4 in LASA Overall QoL (censored)	Week 4	Peg	14	13 (92.9)	10.31 (15.08)	-10.0	3.00	40.0	0.61 (0.45) [-0.22, 1.58]
		SoC	10	10 (100.0)	-1.30 (21.75)	-40.0	0.00	40.0	
	Week 8	Peg	14	12 (85.7)	13.00 (17.98)	-10.0	8.50	40.0	0.76 (0.47) [-0.08, 1.78]
		SoC	10	10 (100.0)	-2.00 (19.99)	-28.0	0.00	40.0	
	Week 12	Peg	14	13 (92.9)	13.69 (18.88)	-20.0	10.00	40.0	0.69 (0.46) [-0.14, 1.67]
		SoC	10	10 (100.0)	0.50 (17.83)	-27.0	0.00	40.0	
	Week 16	Peg	14	13 (92.9)	14.15 (19.15)	-10.0	10.00	40.0	0.62 (0.45) [-0.21, 1.59]
		SoC	10	10 (100.0)	2.00 (18.49)	-27.0	2.50	40.0	
	Week 20	Peg	14	14 (100.0)	14.50 (20.34)	-10.0	8.50	40.0	0.63 (0.45) [-0.19, 1.57]
		SoC	10	10 (100.0)	1.80 (18.41)	-27.0	2.50	40.0	
	Week 26	Peg	14	12 (85.7)	17.42 (18.13)	-10.0	20.00	40.0	1.20 (0.51) [0.34, 2.36]
		SoC	10	9 (90.0)	-2.89 (13.03)	-27.0	0.00	10.0	

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number evaluable values. SD = standard deviation.
 95% CI = 95% confidence interval for Hedges G. Q50 = median. NE = not evaluable.
 Last observation carried forward of the last value observed before escape was used for all patients from the SOC arm who escaped to Peg.
 Source Data: apro, created on: 18SEP2023

Table PT1LTWIT45_SITC: Time to increase of at least 45 points in change from baseline of LASA Total Score (censored) by age
 ITT

Time to increase of at least 45 points in change from baseline of LASA Total Score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LTWIT45_SITC: Time to increase of at least 45 points in change from baseline of LASA Total Score (censored) by age
 ITT

Time to increase of at least 45 points in change from baseline of LASA Total Score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	13 (44.8)	19.07	(4.29, NE)	7.16	(0.92, 55.85)	0.0603	
	SoC	13	1 (7.7)	NE	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LTWIT45_SITC: Time to increase of at least 45 points in change from baseline of LASA Total Score (censored) by age
 ITT

Time to increase of at least 45 points in change from baseline of LASA Total Score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	4 (66.7)	16.43	(8.43, NE)	NE	(NE, NE)	NE	
	SoC	5	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LTWIT45_SITD: Time to increase of at least 45 points in change from baseline of LASA Total Score (censored) by sex
 ITT

Time to increase of at least 45 points in change from baseline of LASA Total Score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LTWIT45_SITD: Time to increase of at least 45 points in change from baseline of LASA Total Score (censored) by sex
 ITT

Time to increase of at least 45 points in change from baseline of LASA Total Score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	8 (42.1)	26.14	(8.14, NE)	NE	(NE, NE)	NE	
	SoC	10	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LTWIT45_SITD: Time to increase of at least 45 points in change from baseline of LASA Total Score (censored) by sex
 ITT

Time to increase of at least 45 points in change from baseline of LASA Total Score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	9 (56.3)	8.43	(4.14, NE)	6.56	(0.73, 59.04)	0.0932	
	SoC	8	1 (12.5)	NE	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LTWIT45_SITA: Time to increase of at least 45 points in change from baseline of LASA Total Score (censored) by number of transfusions previous year
 ITT

Time to increase of at least 45 points in change from baseline of LASA Total Score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LTWIT45_SITA: Time to increase of at least 45 points in change from baseline of LASA Total Score (censored) by number of transfusions previous year
 ITT

Time to increase of at least 45 points in change from baseline of LASA Total Score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	10 (47.6)	26.14	(8.29, NE)	NE	(NE, NE)	NE	
	SoC	8	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LTWIT45_SITA: Time to increase of at least 45 points in change from baseline of LASA Total Score (censored) by number of transfusions previous year
 ITT

Time to increase of at least 45 points in change from baseline of LASA Total Score (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	7 (50.0)	8.14	(4.14, NE)	6.13	(0.75, 50.03)	0.0906	
	SoC	10	1 (10.0)	NE	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWIT15_SITC: Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored) by age
 ITT

Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWIT15_SITC: Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored) by age
 ITT

Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	16 (55.2)	8.36	(4.14, NE)	4.18	(0.95, 18.38)	0.0586	
	SoC	13	2 (15.4)	NE	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWIT15_SITC: Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored) by age
 ITT

Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	4 (66.7)	16.43	(4.57, NE)	NE	(NE, NE)	NE	
	SoC	5	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWIT15_SITD: Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored) by sex
 ITT

Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.8525

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWIT15_SITD: Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored) by sex
 ITT

Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	10 (52.6)	16.43	(4.14, NE)	7.77	(0.92, 65.85)	0.0601	
	SoC	10	1 (10.0)	NE	(8.29, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWIT15_SITD: Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored) by sex
 ITT

Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	10 (62.5)	4.43	(4.14, NE)	5.72	(0.66, 49.55)	0.1134	
	SoC	8	1 (12.5)	NE	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWIT15_SITB: Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored) by region
 ITT

Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWIT15_SITB: Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored) by region
 ITT

Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	9 (75.0)	4.29	(4.14, 8.57)	NE	(NE, NE)	NE	
	SoC	2	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWIT15_SITB: Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored) by region
 ITT

Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	11 (47.8)	20.14	(4.14, NE)	3.23	(0.71, 14.73)	0.1304	
	SoC	16	2 (12.5)	NE	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWIT15_SITA: Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored) by number of transfusions previous year
 ITT

Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								0.8545

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWIT15_SITA: Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored) by number of transfusions previous year
 ITT

Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	12 (57.1)	12.36	(4.14, NE)	4.60	(0.60, 35.53)	0.1432	
	SoC	8	1 (12.5)	NE	(8.29, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWIT15_SITA: Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored) by number of transfusions previous year
 ITT

Time to increase of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	8 (57.1)	14.14	(4.14, NE)	5.74	(0.71, 46.66)	0.1020	
	SoC	10	1 (10.0)	NE	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LDWIT15_SITC: Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by age
 ITT

Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LDWIT15_SITC: Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by age ITT

Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	16 (55.2)	8.14	(4.14, 25.71)	2.29	(0.76, 6.89)	0.1392	
	SoC	13	4 (30.8)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LDWIT15_SITC: Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities
 (censored) by age
 ITT

Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	4 (66.7)	12.14	(4.14, 16.43)	NE	(NE, NE)	NE	
	SoC	5	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LDWIT15_SITD: Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by sex
 ITT

Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.0833

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LDWIT15_SITD: Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by sex ITT

Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	12 (63.2)	8.14	(4.14, NE)	9.25	(1.20, 71.49)	0.0329	*
	SoC	10	1 (10.0)	NE	(16.43, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LDWIT15_SITD: Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by sex
 ITT

Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	8 (50.0)	8.14	(4.14, NE)	1.37	(0.30, 6.19)	0.6837	
	SoC	8	3 (37.5)	8.14	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LDWIT15_SITB: Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by region
 ITT

Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LDWIT15_SITB: Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by region
 ITT

Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	9 (75.0)	4.21	(4.14, 8.14)	NE	(NE, NE)	NE	
	SoC	2	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LDWIT15_SITB: Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by region
 ITT

Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	11 (47.8)	14.29	(8.14, NE)	2.60	(0.80, 8.40)	0.1112	
	SoC	16	4 (25.0)	NE	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LDWIT15_SITA: Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by number of transfusions previous year
 ITT

Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								0.5653

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LDWIT15_SITA: Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by number of transfusions previous year
 ITT

Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	11 (52.4)	12.14	(4.14, NE)	5.33	(0.68, 41.41)	0.1100	
	SoC	8	1 (12.5)	NE	(16.43, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LDWIT15_SITA: Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by number of transfusions previous year
 ITT

Time to increase of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	9 (64.3)	8.14	(4.14, 12.14)	2.64	(0.71, 9.82)	0.1474	
	SoC	10	3 (30.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LQWIT15_SITC: Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored) by age
 ITT

Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Age	Peg								0.6771

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LQWIT15_SITC: Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored) by age
 ITT

Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 60	Peg	29	13 (44.8)	12.14	(4.29, NE)	5.15	(0.65, 40.79)	0.1208	
	SoC	13	1 (7.7)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LQWIT15_SITC: Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored) by age
 ITT

Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 60	Peg	6	5 (83.3)	12.43	(4.14, NE)	1.84	(0.20, 16.62)	0.5872	
	SoC	5	1 (20.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LQWIT15_SITD: Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored) by sex
 ITT

Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								0.8242

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LQWIT15_SITD: Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored) by sex
 ITT

Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	9 (47.4)	20.14	(4.57, NE)	4.29	(0.54, 34.12)	0.1685	
	SoC	10	1 (10.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LQWIT15_SITD: Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored) by sex
 ITT

Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	9 (56.3)	8.43	(4.14, NE)	4.57	(0.51, 40.96)	0.1744	
	SoC	8	1 (12.5)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LQWIT15_SITB: Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored) by region
 ITT

Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								0.3037

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LQWIT15_SITB: Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored) by region
 ITT

Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	8 (66.7)	4.21	(4.14, 8.43)	1.25	(0.14, 10.88)	0.8389	
	SoC	2	1 (50.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LQWIT15_SITB: Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored) by region
 ITT

Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	10 (43.5)	26.14	(8.43, NE)	4.75	(0.59, 37.99)	0.1417	
	SoC	16	1 (6.3)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LQWIT15_SITA: Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored) by number of transfusions previous year
 ITT

Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								0.7781

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LQWIT15_SITA: Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored) by number of transfusions previous year
 ITT

Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	12 (57.1)	12.14	(4.57, NE)	3.03	(0.39, 23.45)	0.2876	
	SoC	8	1 (12.5)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LQWIT15_SITA: Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored) by number of transfusions previous year
 ITT

Time to increase of at least 15 points in change from baseline of LASA Overall QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	6 (42.9)	17.14	(4.14, NE)	4.94	(0.59, 41.20)	0.1401	
	SoC	10	1 (10.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWDT15_SITD: Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored) by sex
 ITT

Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Sex	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWDT15_SITD: Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored) by sex
 ITT

Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Male	Peg	19	4 (21.1)	NE	(NE, NE)	0.26	(0.07, 0.98)	0.0462	*
	SoC	10	6 (60.0)	8.14	(4.14, 16.29)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWDT15_SITD: Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored) by sex
 ITT

Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Female	Peg	16	2 (12.5)	NE	(NE, NE)	NE	(NE, NE)	NE	
	SoC	8	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWDT15_SITB: Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored) by region
 ITT

Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
 time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWDT15_SITB: Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored) by region
 ITT

Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	1 (8.3)	NE	(NE, NE)	NE	(NE, NE)	NE	
	SoC	2	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWDT15_SITB: Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored) by region
ITT

Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	5 (21.7)	NE	(NE, NE)	0.32	(0.10, 1.08)	0.0667	
	SoC	16	6 (37.5)	16.29	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at
time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWDT15_SITA: Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWDT15_SITA: Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	6 (28.6)	NE	(12.14, NE)	0.37	(0.10, 1.35)	0.1325	
	SoC	8	4 (50.0)	12.29	(4.29, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LAWDT15_SITA: Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 15 points in change from baseline of LASA Activity Level (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	0 (0.0)	NE	(NE, NE)	NE	(NE, NE)	NE	
	SoC	10	2 (20.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LDWDT15_SITB: Time to decrease of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by region
 ITT

Time to decrease of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LDWDT15_SITB: Time to decrease of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by region
 ITT

Time to decrease of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	2 (16.7)	NE	(12.14, NE)	NE	(NE, NE)	NE	
	SoC	2	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LDWDT15_SITB: Time to decrease of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by region
 ITT

Time to decrease of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	5 (21.7)	NE	(26.14, NE)	0.21	(0.06, 0.75)	0.0168	*
	SoC	16	6 (37.5)	12.29	(8.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LDWDT15_SITA: Time to decrease of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LDWDT15_SITA: Time to decrease of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
< 4	Peg	21	7 (33.3)	NE	(12.29, NE)	0.28	(0.08, 1.02)	0.0540	
	SoC	8	4 (50.0)	12.14	(4.29, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LDWDT15_SITA: Time to decrease of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored) by number of transfusions previous year
 ITT

Time to decrease of at least 15 points in change from baseline of LASA Ability to Carry Out Daily Activities (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
>= 4	Peg	14	0 (0.0)	NE	(NE, NE)	NE	(NE, NE)	NE	
	SoC	10	2 (20.0)	NE	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factor treatment.
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LQWDT15_SITB: Time to decrease of at least 15 points in change from baseline of LASA Overall QoL (censored) by region
 ITT

Time to decrease of at least 15 points in change from baseline of LASA Overall QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Region	Peg								NE

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LQWDT15_SITB: Time to decrease of at least 15 points in change from baseline of LASA Overall QoL (censored) by region
 ITT

Time to decrease of at least 15 points in change from baseline of LASA Overall QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Latin America	Peg	12	1 (8.3)	NE	(NE, NE)	NE	(NE, NE)	NE	
	SoC	2	0 (0.0)	NE	(NE, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1LQWDT15_SITB: Time to decrease of at least 15 points in change from baseline of LASA Overall QoL (censored) by region
 ITT

Time to decrease of at least 15 points in change from baseline of LASA Overall QoL (censored)				Kaplan-Meier analysis		Cox model			
Subgroup	Treatment	N	nev (%)	Median time (weeks)	95% CI	Hazard Ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	4 (17.4)	NE	(NE, NE)	0.14	(0.04, 0.53)	0.0036	*
	SoC	16	7 (43.8)	12.00	(4.14, NE)				

Note: ITT = Intent-to-Treat Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = number of patients in analysis set. nev (%) = number of patients with at least one event. NE = not evaluable.
 95% CI = 95% confidence interval (median: Brookmeyer/Crowley hazard ratio: coming from Cox model).
 A Cox proportional model was applied with factors treatment and number of PRBC transfusions previous year (<4, >=4).
 Censoring: no event, lost to FU, discontinuation: at last study visit. Escaped SoC: at date of escape.
 No baseline value or no post-baseline value or if not treated or could not develop respective event due to floor/ceiling effect: at time of first drug intake.* = significant treatment effect. IA = p-value of interaction term.
 Source Data: apro_tte, created on: 22SEP2023

Table PT1A_SSNC: TEAEs during RCP by age
 SAF

TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Age	Peg									0.6600

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1A_SSNC: TEAEs during RCP by age
 SAF

TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
< 60	Peg	29	29 (100.0)	14.2	23 (79.3)	1.62	0.67	(0.32, 1.41)	0.2927	
	SoC	13	13 (100.0)	4.1	10 (76.9)	2.41				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1A_SSNC: TEAEs during RCP by age
 SAF

TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
>= 60	Peg	6	6 (100.0)	3.1	5 (83.3)	1.63	1.00	(0.19, 5.15)	0.9987	
	SoC	5	5 (100.0)	1.2	2 (40.0)	1.63				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1A_SSND: TEAEs during RCP by sex
 SAF

TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Sex	Peg									0.4939

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1A_SSND: TEAEs during RCP by sex
 SAF

TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Male	Peg	19	19 (100.0)	9.6	16 (84.2)	1.66	0.87	(0.36, 2.11)	0.7565	
	SoC	10	10 (100.0)	3.7	7 (70.0)	1.91				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1A_SSND: TEAEs during RCP by sex
 SAF

TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Female	Peg	16	16 (100.0)	7.6	12 (75.0)	1.57	0.54	(0.19, 1.52)	0.2423	
	SoC	8	8 (100.0)	1.7	5 (62.5)	2.93				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1A_SSNB: TEAEs during RCP by region
 SAF

TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Region	Peg									0.1693

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1A_SSNB: TEAEs during RCP by region
 SAF

TEAEs during RCP				Negative binomial model						
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Latin America	Peg	12	12 (100.0)	5.7	10 (83.3)	1.76	0.21	(0.05, 0.97)	0.0454	*
	SoC	2	2 (100.0)	0.2	2 (100.0)	8.30				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1A_SSNB: TEAEs during RCP by region
 SAF

TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	23 (100.0)	11.6	18 (78.3)	1.55	0.80	(0.37, 1.73)	0.5642	
	SoC	16	16 (100.0)	5.1	10 (62.5)	1.95				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1A_SSNA: TEAEs during RCP by number of transfusions previous year
 SAF

TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg									0.9209

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1A_SSNA: TEAEs during RCP by number of transfusions previous year
 SAF

TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
< 4	Peg	21	21 (100.0)	10.2	17 (81.0)	1.67	0.70	(0.29, 1.69)	0.4311	
	SoC	8	8 (100.0)	2.9	7 (87.5)	2.38				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1A_SSNA: TEAEs during RCP by number of transfusions previous year
 SAF

TEAEs during RCP				Negative binomial model						
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
>= 4	Peg	14	14 (100.0)	7.1	11 (78.6)	1.55	0.75	(0.26, 2.17)	0.5987	
	SoC	10	10 (100.0)	2.4	5 (50.0)	2.06				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AM_SSNC: Mild TEAEs during RCP by age
 SAF

Mild TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Age	Peg									0.4842

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AM_SSNC: Mild TEAEs during RCP by age
 SAF

Mild TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
< 60	Peg	29	29 (100.0)	14.2	22 (75.9)	1.55	0.92	(0.39, 2.15)	0.8423	
	SoC	13	13 (100.0)	4.1	7 (53.8)	1.69				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AM_SSNC: Mild TEAEs during RCP by age
 SAF

Mild TEAEs during RCP				Negative binomial model						
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
>= 60	Peg	6	6 (100.0)	3.1	5 (83.3)	1.63	2.00	(0.23, 17.10)	0.5277	
	SoC	5	5 (100.0)	1.2	1 (20.0)	0.82				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AM_SSND: Mild TEAEs during RCP by sex
 SAF

Mild TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Sex	Peg									0.3521

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AM_SSND: Mild TEAEs during RCP by sex
 SAF

Mild TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Male	Peg	19	19 (100.0)	9.6	15 (78.9)	1.56	1.43	(0.47, 4.30)	0.5286	
	SoC	10	10 (100.0)	3.7	4 (40.0)	1.09				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AM_SSND: Mild TEAEs during RCP by sex
 SAF

Mild TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Female	Peg	16	16 (100.0)	7.6	12 (75.0)	1.57	0.67	(0.22, 2.08)	0.4893	
	SoC	8	8 (100.0)	1.7	4 (50.0)	2.35				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AM_SSNB: Mild TEAEs during RCP by region
 SAF

Mild TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Region	Peg									0.4579

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AM_SSNB: Mild TEAEs during RCP by region
 SAF

Mild TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Latin America	Peg	12	12 (100.0)	5.7	10 (83.3)	1.76	0.42	(0.05, 3.32)	0.4140	
	SoC	2	2 (100.0)	0.2	1 (50.0)	4.15				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AM_SSNB: Mild TEAEs during RCP by region
 SAF

Mild TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	23 (100.0)	11.6	17 (73.9)	1.47	1.07	(0.45, 2.59)	0.8724	
	SoC	16	16 (100.0)	5.1	7 (43.8)	1.37				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AM_SSNA: Mild TEAEs during RCP by number of transfusions previous year
 SAF

Mild TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg									0.8577

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AM_SSNA: Mild TEAEs during RCP by number of transfusions previous year
 SAF

Mild TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
< 4	Peg	21	21 (100.0)	10.2	17 (81.0)	1.67	0.98	(0.36, 2.66)	0.9732	
	SoC	8	8 (100.0)	2.9	5 (62.5)	1.70				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AM_SSNA: Mild TEAEs during RCP by number of transfusions previous year
 SAF

Mild TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
>= 4	Peg	14	14 (100.0)	7.1	10 (71.4)	1.41	1.14	(0.31, 4.14)	0.8415	
	SoC	10	10 (100.0)	2.4	3 (30.0)	1.24				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AI_SSNC: Moderate TEAEs during RCP by age
 SAF

Moderate TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Age	Peg									0.9462

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AI_SSNC: Moderate TEAEs during RCP by age
 SAF

Moderate TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
< 60	Peg	29	29 (100.0)	14.2	12 (41.4)	0.85	0.88	(0.28, 2.71)	0.8180	
	SoC	13	13 (100.0)	4.1	4 (30.8)	0.97				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AI_SSNC: Moderate TEAEs during RCP by age
 SAF

Moderate TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
>= 60	Peg	6	6 (100.0)	3.1	2 (33.3)	0.65	0.80	(0.07, 8.81)	0.8546	
	SoC	5	5 (100.0)	1.2	1 (20.0)	0.82				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AI_SSND: Moderate TEAEs during RCP by sex
 SAF

Moderate TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Sex	Peg									0.3481

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AI_SSND: Moderate TEAEs during RCP by sex
 SAF

Moderate TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Male	Peg	19	19 (100.0)	9.6	10 (52.6)	1.04	1.27	(0.35, 4.60)	0.7191	
	SoC	10	10 (100.0)	3.7	3 (30.0)	0.82				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AI_SSND: Moderate TEAEs during RCP by sex
 SAF

Moderate TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Female	Peg	16	16 (100.0)	7.6	4 (25.0)	0.52	0.45	(0.08, 2.44)	0.3528	
	SoC	8	8 (100.0)	1.7	2 (25.0)	1.17				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AI_SSNB: Moderate TEAEs during RCP by region
 SAF

Moderate TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Region	Peg									0.2691

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AI_SSNB: Moderate TEAEs during RCP by region
 SAF

Moderate TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Latin America	Peg	12	12 (100.0)	5.7	5 (41.7)	0.88	0.21	(0.02, 1.82)	0.1571	
	SoC	2	2 (100.0)	0.2	1 (50.0)	4.15				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AI_SSNB: Moderate TEAEs during RCP by region
 SAF

Moderate TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	23 (100.0)	11.6	9 (39.1)	0.78	1.00	(0.31, 3.23)	0.9943	
	SoC	16	16 (100.0)	5.1	4 (25.0)	0.78				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AI_SSNA: Moderate TEAEs during RCP by number of transfusions previous year
 SAF

Moderate TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg									NE

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AI_SSNA: Moderate TEAEs during RCP by number of transfusions previous year
 SAF

Moderate TEAEs during RCP							Negative binomial model			
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
< 4	Peg	21	21 (100.0)	10.2	6 (28.6)	0.59	0.87	(0.18, 4.30)	0.8617	
	SoC	8	8 (100.0)	2.9	2 (25.0)	0.68				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AI_SSNA: Moderate TEAEs during RCP by number of transfusions previous year
 SAF

Moderate TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
>= 4	Peg	14	14 (100.0)	7.1	8 (57.1)	1.13	0.91	(0.24, 3.44)	0.8925	
	SoC	10	10 (100.0)	2.4	3 (30.0)	1.24				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AA_SSNC: Non-disease related TEAEs during RCP by age
 SAF

Non-disease related TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Age	Peg									0.9656

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AA_SSNC: Non-disease related TEAEs during RCP by age
 SAF

Non-disease related TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
< 60	Peg	29	29 (100.0)	14.2	23 (79.3)	1.62	0.96	(0.41, 2.23)	0.9227	
	SoC	13	13 (100.0)	4.1	7 (53.8)	1.69				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AA_SSNC: Non-disease related TEAEs during RCP by age
 SAF

Non-disease related TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
>= 60	Peg	6	6 (100.0)	3.1	5 (83.3)	1.63	1.00	(0.19, 5.15)	0.9987	
	SoC	5	5 (100.0)	1.2	2 (40.0)	1.63				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AA_SSND: Non-disease related TEAEs during RCP by sex
 SAF

Non-disease related TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Sex	Peg									0.1762

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AA_SSND: Non-disease related TEAEs during RCP by sex
 SAF

Non-disease related TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Male	Peg	19	19 (100.0)	9.6	16 (84.2)	1.66	1.52	(0.51, 4.55)	0.4534	
	SoC	10	10 (100.0)	3.7	4 (40.0)	1.09				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AA_SSND: Non-disease related TEAEs during RCP by sex
 SAF

Non-disease related TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Female	Peg	16	16 (100.0)	7.6	12 (75.0)	1.57	0.54	(0.19, 1.52)	0.2423	
	SoC	8	8 (100.0)	1.7	5 (62.5)	2.93				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AA_SSNB: Non-disease related TEAEs during RCP by region
 SAF

Non-disease related TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Region	Peg									NE

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AA_SSNB: Non-disease related TEAEs during RCP by region
 SAF

Non-disease related TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Latin America	Peg	12	12 (100.0)	5.7	10 (83.3)	1.76	NE	(NE, NE)	NE	
	SoC	2	2 (100.0)	0.2	0 (0.0)	0.00				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AA_SSNB: Non-disease related TEAEs during RCP by region
 SAF

Non-disease related TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Asia Pacific	Peg	23	23 (100.0)	11.6	18 (78.3)	1.55	0.89	(0.40, 1.97)	0.7650	
	SoC	16	16 (100.0)	5.1	9 (56.3)	1.76				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AA_SSNA: Non-disease related TEAEs during RCP by number of transfusions previous year
 SAF

Non-disease related TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Number of transfusions in the previous year	Peg									0.9550

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AA_SSNA: Non-disease related TEAEs during RCP by number of transfusions previous year
 SAF

Non-disease related TEAEs during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
< 4	Peg	21	21 (100.0)	10.2	17 (81.0)	1.67	0.98	(0.36, 2.66)	0.9732	
	SoC	8	8 (100.0)	2.9	5 (62.5)	1.70				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AA_SSNA: Non-disease related TEAEs during RCP by number of transfusions previous year
 SAF

Non-disease related TEAEs during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
>= 4	Peg	14	14 (100.0)	7.1	11 (78.6)	1.55	0.94	(0.30, 2.96)	0.9171	
	SoC	10	10 (100.0)	2.4	4 (40.0)	1.65				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). TEAE = treatment emergent adverse event.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AF_SSNC: AESI Infections and Infestations during RCP by age
 SAF

AESI Infections and Infestations during RCP					Negative binomial model					
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
Age	Peg									0.8738

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). AESI = adverse event of special interest.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AF_SSNC: AESI Infections and Infestations during RCP by age
 SAF

AESI Infections and Infestations during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
< 60	Peg	29	29 (100.0)	14.2	7 (24.1)	0.49	0.51	(0.15, 1.74)	0.2838	
	SoC	13	13 (100.0)	4.1	4 (30.8)	0.97				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). AESI = adverse event of special interest.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AF_SSNC: AESI Infections and Infestations during RCP by age
 SAF

AESI Infections and Infestations during RCP						Negative binomial model				
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR	Rate ratio	95% CI	p-value	IA
>= 60	Peg	6	6 (100.0)	3.1	1 (16.7)	0.33	0.40	(0.02, 6.39)	0.5164	
	SoC	5	5 (100.0)	1.2	1 (20.0)	0.82				

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). AESI = adverse event of special interest.
 95% CI = 95% confidence interval. * = significant treatment effect.
 CI, p-value, and rate ratio = negative binomial regression model, offset = observed individual duration of treatment period.
 IA = p-value of interaction term.
 Source Data: aae, created on: 01SEP2023

Table PT1AL_SSNC: AESI Injection site reaction during RCP by age
 SAF

AESI Injection site reaction during RCP						
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR (95% CI)
< 60	Peg	29	29 (100.0)	14.2	10 (34.5)	0.70 (0.38, 1.31)

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). AESI = adverse event of special interest.
 Source Data: aae, created on: 01SEP2023

Table PT1AL_SSNC: AESI Injection site reaction during RCP by age
 SAF

AESI Injection site reaction during RCP						
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR (95% CI)
>= 60	Peg	6	6 (100.0)	3.1	1 (16.7)	0.33 (0.05, 2.32)

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). AESI = adverse event of special interest.
 Source Data: aae, created on: 01SEP2023

Table PT1ALM_SSNC: Mild AESI Injection site reaction during RCP by age
 SAF

Mild AESI Injection site reaction during RCP						
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR (95% CI)
< 60	Peg	29	29 (100.0)	14.2	10 (34.5)	0.70 (0.38, 1.31)

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). AESI = adverse event of special interest.
 Source Data: aae, created on: 01SEP2023

Table PT1ALM_SSNC: Mild AESI Injection site reaction during RCP by age
 SAF

Mild AESI Injection site reaction during RCP						
Subgroup	Treatment	N	nval (%)	Time at risk (years)	n (%)	EAIR (95% CI)
>= 60	Peg	6	6 (100.0)	3.1	1 (16.7)	0.33 (0.05, 2.32)

Note: SAF = Safety Set. Peg = Pegcetacoplan. SoC = Standard of care.
 N = total number of patients in analysis set. n = number of patients with events. nval = number of considered patients.
 EAIR: exposure adjusted incidence rate (yearly rate). AESI = adverse event of special interest.
 Source Data: aae, created on: 01SEP2023