*Momelotinib (Omjjara)*

GlaxoSmithKline GmbH & Co. KG

# Anhang 4-H zu Modul 4 A

*Behandlung von krankheitsbedingter Splenomegalie oder Symptomen bei erwachsenen Patienten mit moderater bis schwerer Anämie, die an primärer Myelofibrose, Post-Polycythaemia Vera-Myelofibrose oder Post-Essentieller Thrombozythämie-Myelofibrose erkrankt sind, und die nicht mit einem Januskinase (JAK)-Inhibitor vorbehandelt sind oder die mit Ruxolitinib behandelt wurden*

Stand: 15.02.2024

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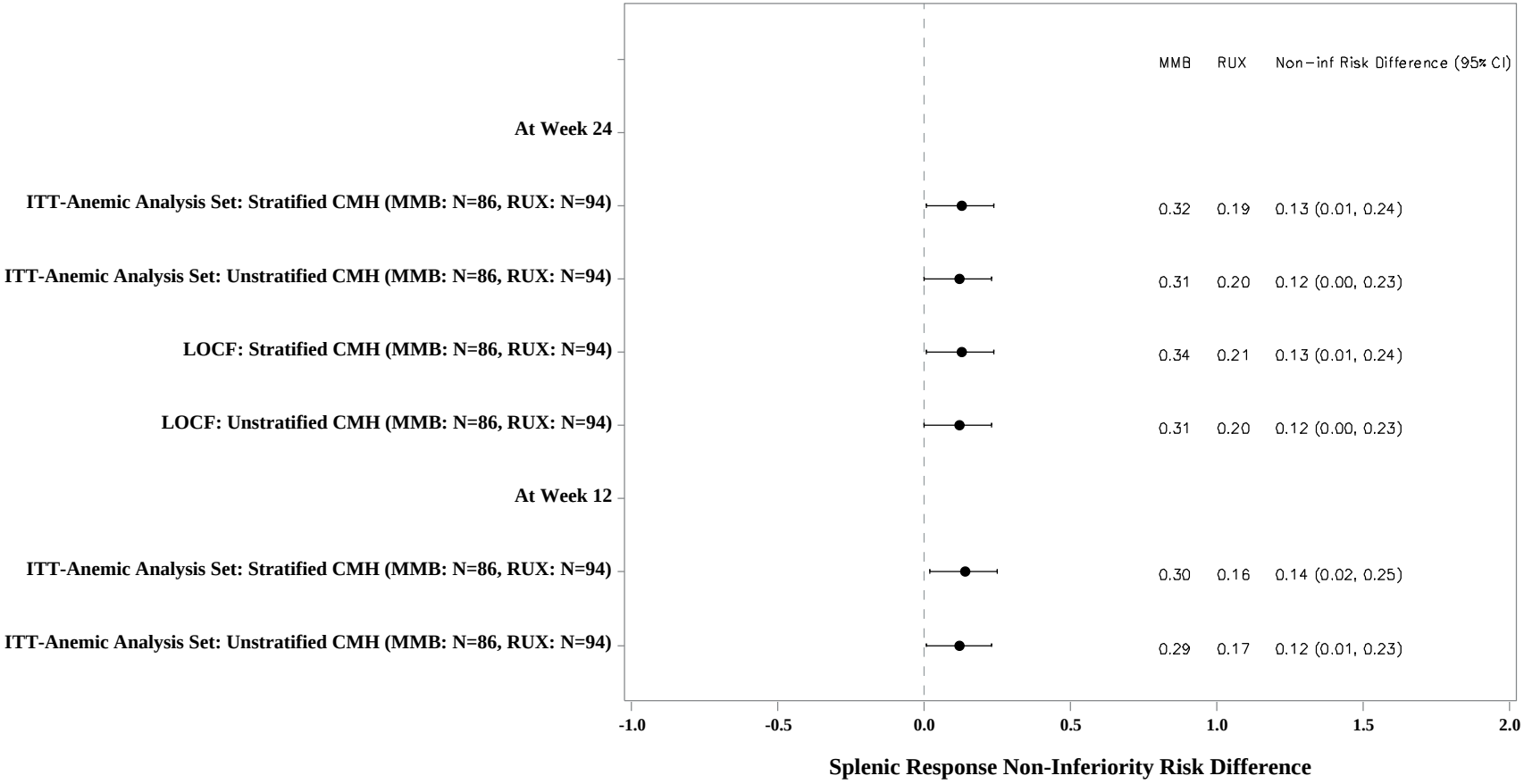
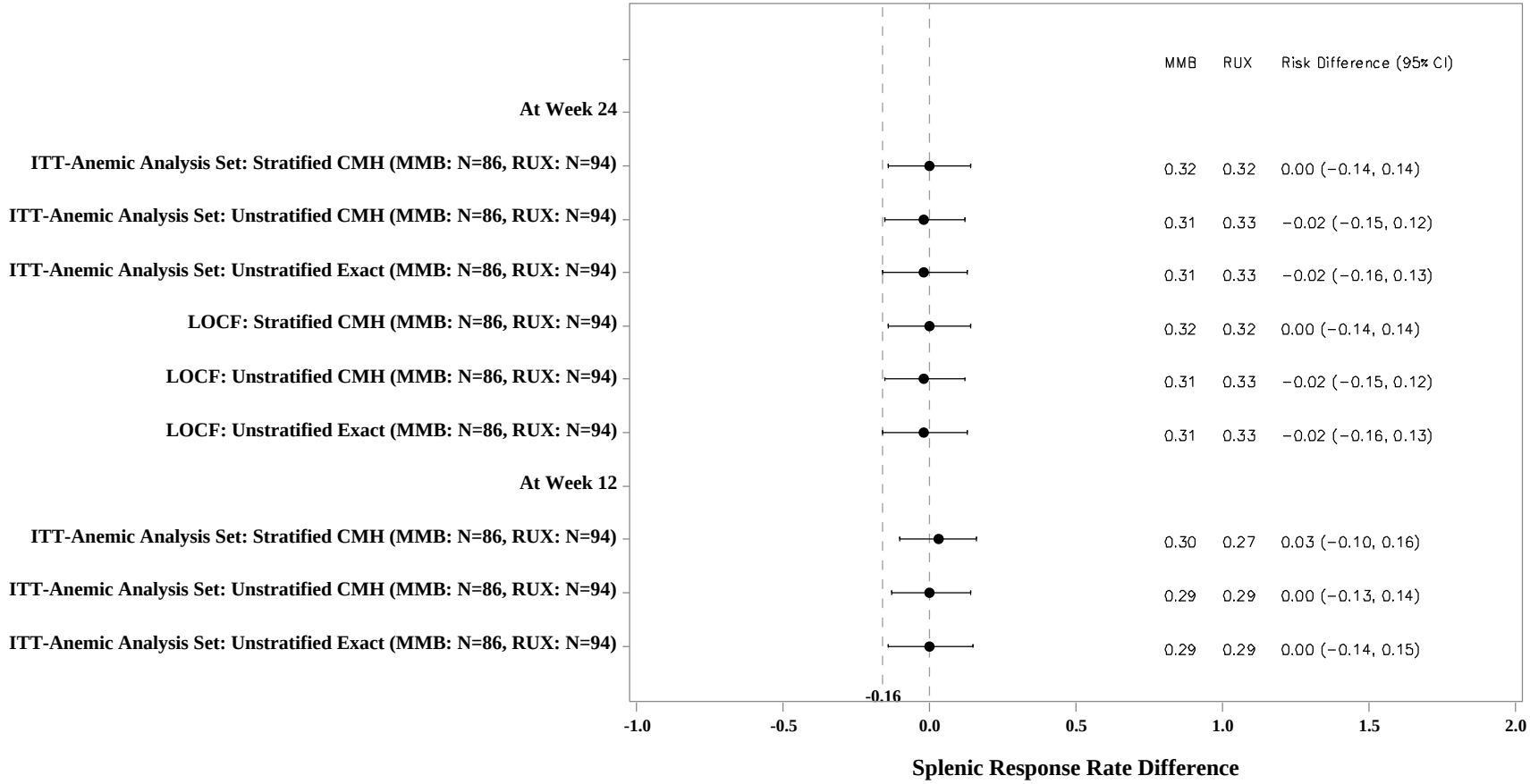
Figure 2.0201: Forest plot of Primary and Sensitivity Analysis of Splenic Response Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic Analysis Set
Noninferiority




ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
To the right of the reference line favors MMB, to the left favors RUX.
Non-inferiority risk difference calculated on a factor of 0.6.
CMH = Cochran-Mantel-Haenszel; LOCF = Last Observation Carried Forward; CI= Confidence Interval

Data Extracted: CRF data: 01JUL2019
Source: g-srr-forest.sas V.03.05 Output file: g-srr-forest.pdf 30AUG2023:12:27

Figure 2.0201: Forest plot of Primary and Sensitivity Analysis of Splenic Response Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic Analysis Set
Superiority and Fixed Margin Noninferiority

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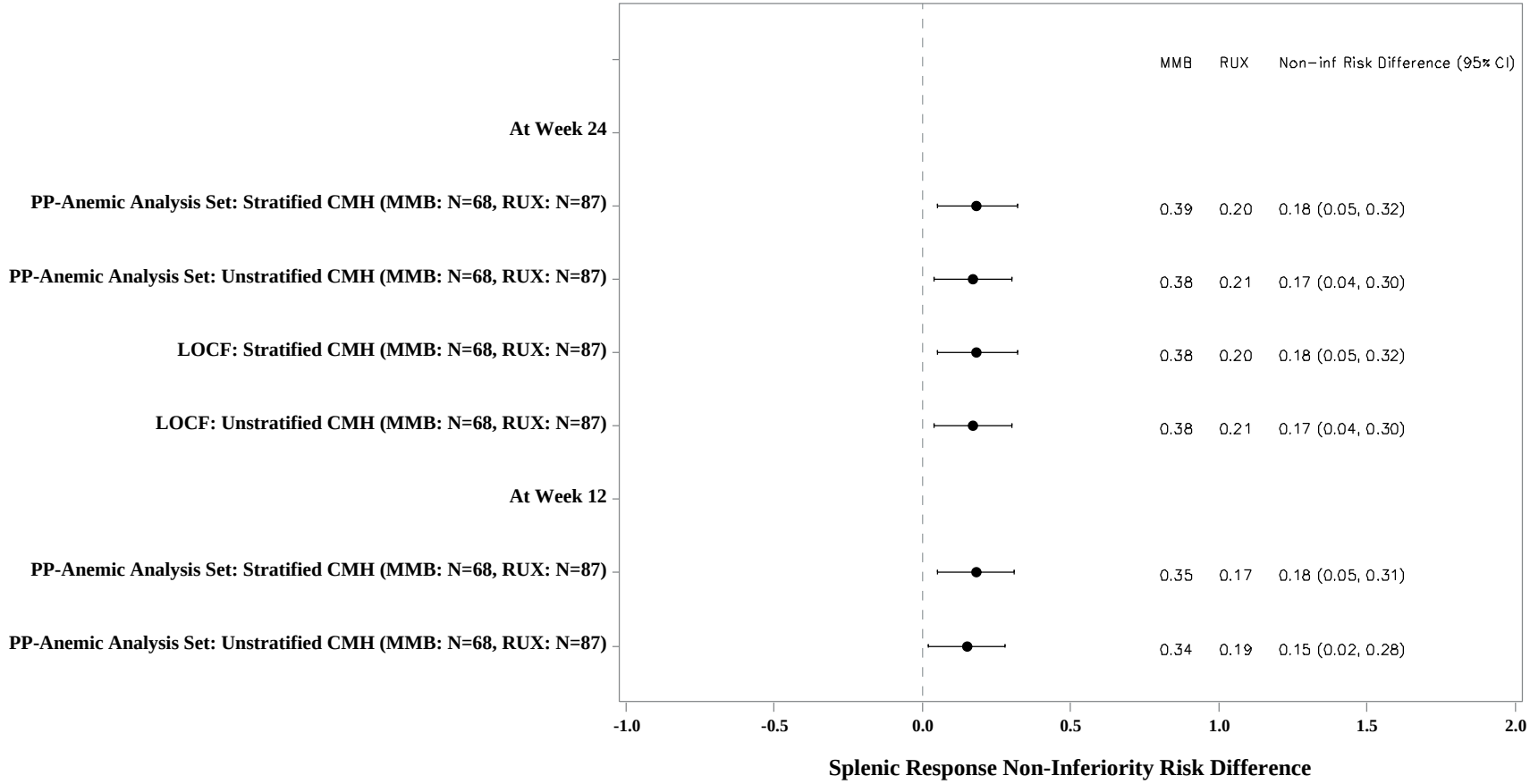
CMH = Cochran-Mantel-Haenszel; LOCF = Last Observation Carried Forward; CI= Confidence Interval

Data Extracted: CRF data: 01JUL2019
Source: g-srr-forest.sas V.03.05 Output file: g-srr-forest.pdf 30AUG2023:12:27

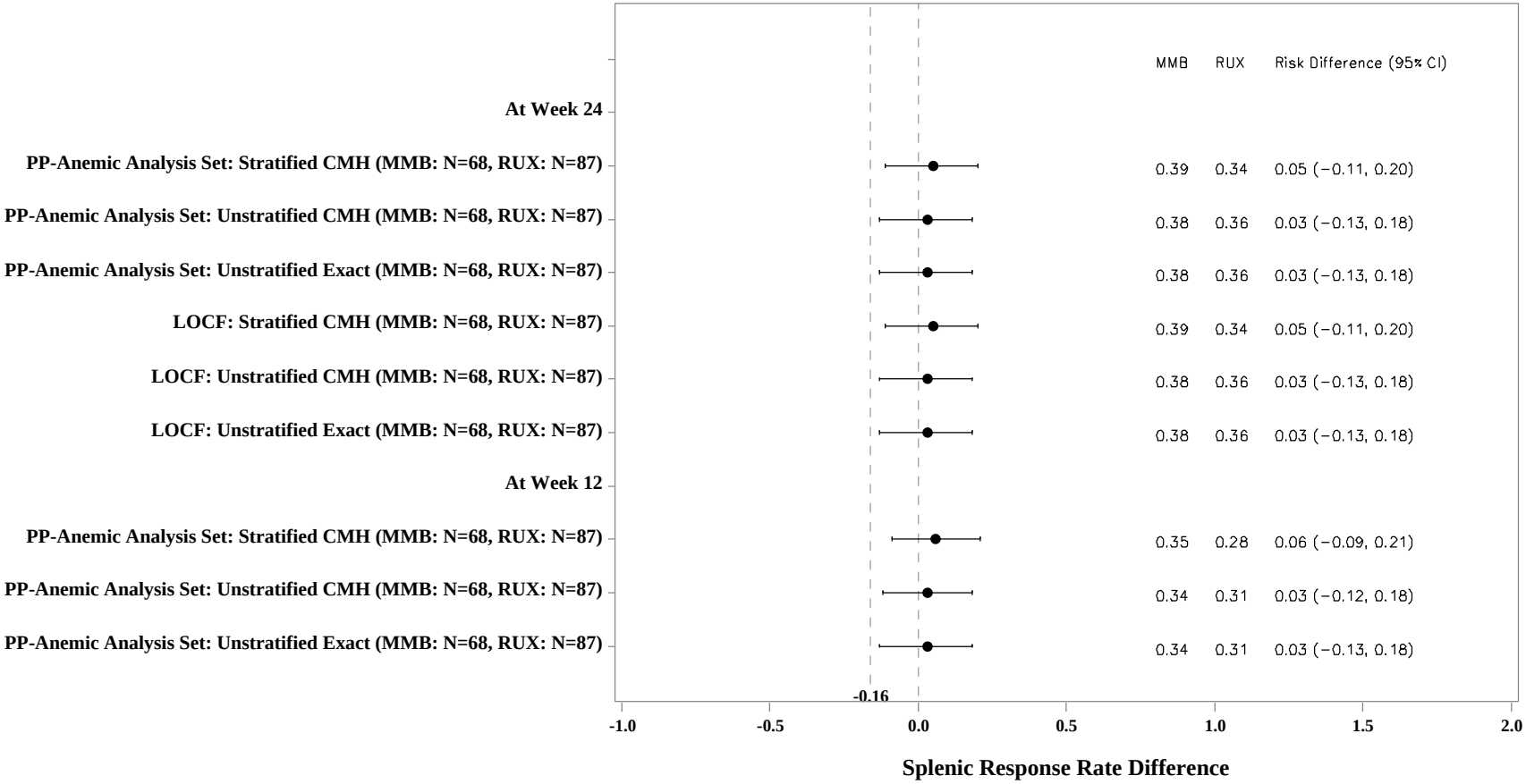
Figure 2.0202: Forest plot of Primary and Sensitivity Analysis of Splenic Response Rate at Week 24 and Week 12
Double-Blind Phase
PP-Anemic Analysis Set
Noninferiority




PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
To the right of the reference line favors MMB, to the left favors RUX.
Non-inferiority risk difference calculated on a factor of 0.6.
CMH = Cochran-Mantel-Haenszel; LOCF = Last Observation Carried Forward; PP=Per-Protocol; CI= Confidence Interval

Data Extracted: CRF data: 01JUL2019
Source: g-srr-forest.sas V.03.05 Output file: g-srr-forest-pp.pdf 30AUG2023:12:27

Figure 2.0202: Forest plot of Primary and Sensitivity Analysis of Splenic Response Rate at Week 24 and Week 12
Double-Blind Phase
PP-Anemic Analysis Set
Superiority and Fixed Margin Noninferiority




PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
To the right of the reference line favors MMB, to the left favors RUX.
Non-inferiority risk difference calculated on a factor of 0.6.
CMH = Cochran-Mantel-Haenszel; LOCF = Last Observation Carried Forward; PP=Per-Protocol; CI= Confidence Interval

Data Extracted: CRF data: 01JUL2019
Source: g-srr-forest.sas V.03.05 Output file: g-srr-forest-pp.pdf 30AUG2023:12:27

Table 2.0301: Sensitivity Analysis of Splenic Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| All Stratum Combined | | | |
| Splenic Response Rate at Week 24 | | | |
| Responder at Week 24, n(%) | 27 ( 31.4%) | 31 ( 33.0%) | 58 ( 32.2%) |
| Responder from LOCF, n(%) | 0 | 0 | 0 |
| Noninferior Proportion Difference - Stratified CMH Method (95% CI) | 0.13 (0.01, 0.24) | | |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.12 (0.00, 0.23) | | |
| Superior Proportion Difference - Stratified CMH Method (95% CI) | 0.00 (-0.14, 0.14) | | |
| p-value | 0.98 | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.02 (-0.15, 0.12) | | |
| p-value | 0.82 | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.02 (-0.16, 0.13) | | |
| p-value | 0.87 | | |
| Non-Responder, n(%) | 59 ( 68.6%) | 63 ( 67.0%) | 122 ( 67.8%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 12 ( 14.0%) | 3 ( 3.2%) | 15 ( 8.3%) |
| >0% Spleen Volume increase at Week 24 | 10 ( 11.6%) | 9 ( 9.6%) | 19 ( 10.6%) |
| <35% Spleen Volume reduction at Week 24 | 47 ( 54.7%) | 60 ( 63.8%) | 107 ( 59.4%) |
| Last participation date < Day 141 in DB Phase | 12 ( 14.0%) | 4 ( 4.3%) | 16 ( 8.9%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel

Data Extracted: CRF data: 01JUL2019
Source: t-srr24-sen.sas V.03.05 Output file: t-sen-locf-srr24.pdf 29AUG2023: 9:45

Table 2.0301: Sensitivity Analysis of Splenic Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Platelet Count < 100X10E9/L | 10 | 9 | 19 |
| Splenic Response Rate at Week 24 | | | |
| Responder at Week 24, n(%) | 5 ( 50.0%) | 0 | 5 ( 26.3%) |
| Responder from LOCF, n(%) | 0 | 0 | 0 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.50 (0.16, 0.84) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.50 (0.14, 0.86) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.50 (0.08, 0.82) | | |
| Non-Responder, n(%) | 5 ( 50.0%) | 9 (100.0%) | 14 ( 73.7%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 1 ( 10.0%) | 1 ( 11.1%) | 2 ( 10.5%) |
| >0% Spleen Volume increase at Week 24 | 1 ( 10.0%) | 2 ( 22.2%) | 3 ( 15.8%) |
| <35% Spleen Volume reduction at Week 24 | 4 ( 40.0%) | 8 ( 88.9%) | 12 ( 63.2%) |
| Last participation date < Day 141 in DB Phase | 1 ( 10.0%) | 2 ( 22.2%) | 3 ( 15.8%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel

Data Extracted: CRF data: 01JUL2019
Source: t-srr24-sen.sas V.03.05  Output file: t-sen-locf-srr24.pdf 29AUG2023: 9:45

Table 2.0301: Sensitivity Analysis of Splenic Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 20 | 18 | 38 |
| Splenic Response Rate at Week 24 | | | |
| Responder at Week 24, n(%) | 5 ( 25.0%) | 4 ( 22.2%) | 9 ( 23.7%) |
| Responder from LOCF, n(%) | 0 | 0 | 0 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.12 (-0.12, 0.35) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.03 (-0.25, 0.31) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.03 (-0.29, 0.34) | | |
| Non-Responder, n(%) | 15 ( 75.0%) | 14 ( 77.8%) | 29 ( 76.3%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 4 ( 20.0%) | 2 ( 11.1%) | 6 ( 15.8%) |
| >0% Spleen Volume increase at Week 24 | 3 ( 15.0%) | 3 ( 16.7%) | 6 ( 15.8%) |
| <35% Spleen Volume reduction at Week 24 | 11 ( 55.0%) | 12 ( 66.7%) | 23 ( 60.5%) |
| Last participation date < Day 141 in DB Phase | 4 ( 20.0%) | 2 ( 11.1%) | 6 ( 15.8%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel

Data Extracted: CRF data: 01JUL2019
Source: t-srr24-sen.sas V.03.05  Output file: t-sen-locf-srr24.pdf 29AUG2023: 9:45

Table 2.0301: Sensitivity Analysis of Splenic Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence Yes and Platelet Count > 200X10E9/L | 19 | 16 | 35 |
| Splenic Response Rate at Week 24 | | | |
| Responder at Week 24, n(%) | 2 ( 10.5%) | 8 ( 50.0%) | 10 ( 28.6%) |
| Responder from LOCF, n(%) | 0 | 0 | 0 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | -0.19 (-0.41, 0.02) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.39 (-0.69, -0.10) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.39 (-0.66, -0.06) | | |
| Non-Responder, n(%) | 17 ( 89.5%) | 8 ( 50.0%) | 25 ( 71.4%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 2 ( 10.5%) | 0 | 2 ( 5.7%) |
| >0% Spleen Volume increase at Week 24 | 3 ( 15.8%) | 1 ( 6.3%) | 4 ( 11.4%) |
| <35% Spleen Volume reduction at Week 24 | 15 ( 78.9%) | 8 ( 50.0%) | 23 ( 65.7%) |
| Last participation date < Day 141 in DB Phase | 3 ( 15.8%) | 0 | 3 ( 8.6%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel

Data Extracted: CRF data: 01JUL2019
Source: t-srr24-sen.sas V.03.05 Output file: t-sen-locf-srr24.pdf 29AUG2023: 9:45

Table 2.0301: Sensitivity Analysis of Splenic Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Platelet Count < 100X10E9/L | 3 | 4 | 7 |
| Splenic Response Rate at Week 24 | | | |
| Responder at Week 24, n(%) | 1 ( 33.3%) | 0 | 1 ( 14.3%) |
| Responder from LOCF, n(%) | 0 | 0 | 0 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.33 (-0.37, 1.03) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.33 (-0.42, 1.09) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.33 (-0.43, 0.91) | | |
| Non-Responder, n(%) | 2 ( 66.7%) | 4 (100.0%) | 6 ( 85.7%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 0 | 0 | 0 |
| >0% Spleen Volume increase at Week 24 | 1 ( 33.3%) | 1 ( 25.0%) | 2 ( 28.6%) |
| <35% Spleen Volume reduction at Week 24 | 2 ( 66.7%) | 4 (100.0%) | 6 ( 85.7%) |
| Last participation date < Day 141 in DB Phase | 0 | 0 | 0 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel

Data Extracted: CRF data: 01JUL2019
Source: t-srr24-sen.sas V.03.05  Output file: t-sen-locf-srr24.pdf 29AUG2023: 9:45

Table 2.0301: Sensitivity Analysis of Splenic Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 5 | | | |
| Transfusion Dependence No and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 16 | 16 | 32 |
| Splenic Response Rate at Week 24 | | | |
| Responder at Week 24, n(%) | 8 ( 50.0%) | 4 ( 25.0%) | 12 ( 37.5%) |
| Responder from LOCF, n(%) | 0 | 0 | 0 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.35 (0.06, 0.64) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.25 (-0.09, 0.59) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.25 (-0.13, 0.58) | | |
| Non-Responder, n(%) | 8 ( 50.0%) | 12 ( 75.0%) | 20 ( 62.5%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 1 ( 6.3%) | 0 | 1 ( 3.1%) |
| >0% Spleen Volume increase at Week 24 | 1 ( 6.3%) | 0 | 1 ( 3.1%) |
| <35% Spleen Volume reduction at Week 24 | 7 ( 43.8%) | 12 ( 75.0%) | 19 ( 59.4%) |
| Last participation date < Day 141 in DB Phase | 2 ( 12.5%) | 0 | 2 ( 6.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel

Data Extracted: CRF data: 01JUL2019
Source: t-srr24-sen.sas V.03.05 Output file: t-sen-locf-srr24.pdf 29AUG2023: 9:45

Table 2.0301: Sensitivity Analysis of Splenic Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 6 | | | |
| Transfusion Dependence No and Platelet Count > 200X10E9/L | 18 | 31 | 49 |
| Splenic Response Rate at Week 24 | | | |
| Responder at Week 24, n(%) | 6 ( 33.3%) | 15 ( 48.4%) | 21 ( 42.9%) |
| Responder from LOCF, n(%) | 0 | 0 | 0 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.04 (-0.21, 0.29) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.15 (-0.44, 0.14) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.15 (-0.42, 0.14) | | |
| Non-Responder, n(%) | 12 ( 66.7%) | 16 ( 51.6%) | 28 ( 57.1%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 4 ( 22.2%) | 0 | 4 ( 8.2%) |
| >0% Spleen Volume increase at Week 24 | 1 ( 5.6%) | 2 ( 6.5%) | 3 ( 6.1%) |
| <35% Spleen Volume reduction at Week 24 | 8 ( 44.4%) | 16 ( 51.6%) | 24 ( 49.0%) |
| Last participation date < Day 141 in DB Phase | 2 ( 11.1%) | 0 | 2 ( 4.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel

Data Extracted: CRF data: 01JUL2019
Source: t-srr24-sen.sas V.03.05 Output file: t-sen-locf-srr24.pdf 29AUG2023: 9:45

Table 2.0302: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| All Stratum Combined | | | |
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 26 ( 38.2%) | 31 ( 35.6%) | 57 ( 36.8%) |
| 95% Exact CI | 0.2671, 0.5082 | 0.2565, 0.4662 | 0.2918, 0.4488 |
| Noninferior Proportion Difference - Stratified CMH Method (95% CI) | 0.18 (0.05, 0.32) | | |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.17 (0.04, 0.30) | | |
| Superior Proportion Difference - Stratified CMH Method (95% CI) | 0.05 (-0.11, 0.20) | | |
| p-value | 0.54 | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.03 (-0.13, 0.18) | | |
| p-value | 0.74 | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.03 (-0.13, 0.18) | | |
| p-value | 0.74 | | |
| Non-Responder, n(%) | 42 ( 61.8%) | 56 ( 64.4%) | 98 ( 63.2%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 3 ( 4.4%) | 4 ( 4.6%) | 7 ( 4.5%) |
| >0% Spleen Volume increase at Week 24 | 8 ( 11.8%) | 7 ( 8.0%) | 15 ( 9.7%) |
| <35% Spleen Volume reduction at Week 24 | 39 ( 57.4%) | 52 ( 59.8%) | 91 ( 58.7%) |
| Last participation date < Day 141 in DB Phase | 0 | 0 | 0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol

Data Extracted: CRF data: 01JUL2019
Source: t-srr24-sen.sas V.03.05 Output file: t-sen-ppt-srr24.pdf 29AUG2023: 9:44

Table 2.0302: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 23 ( 33.8%) | 27 ( 31.0%) | 50 ( 32.3%) |
| 95% Exact CI | 0.2279, 0.4632 | 0.2155, 0.4186 | 0.2498, 0.4023 |
| Noninferior Proportion Difference - Stratified CMH Method (95% CI) | 0.18 (0.05, 0.31) | | |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.15 (0.02, 0.28) | | |
| Superior Proportion Difference - Stratified CMH Method (95% CI) | 0.06 (-0.09, 0.21) | | |
| p-value | 0.41 | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.03 (-0.12, 0.18) | | |
| p-value | 0.72 | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.03 (-0.13, 0.18) | | |
| p-value | 0.73 | | |
| Non-Responder, n(%) | 45 ( 66.2%) | 60 ( 69.0%) | 105 ( 67.7%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 12 not available | 2 ( 2.9%) | 2 ( 2.3%) | 4 ( 2.6%) |
| >0% Spleen Volume increase at Week 12 | 5 ( 7.4%) | 8 ( 9.2%) | 13 ( 8.4%) |
| <35% Spleen Volume reduction at Week 12 | 43 ( 63.2%) | 58 ( 66.7%) | 101 ( 65.2%) |
| Last participation date < Day 57 in DB Phase | 0 | 0 | 0 |
| Splenic Response Rate at Any Timepoint in DB Phase | | | |
| Responder, n(%) | 29 ( 42.6%) | 36 ( 41.4%) | 65 ( 41.9%) |
| 95% Exact CI | 0.3072, 0.5523 | 0.3092, 0.5245 | 0.3407, 0.5012 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol

Data Extracted: CRF data: 01JUL2019
Source: t-srr24-sen.sas V.03.05 Output file: t-sen-ppt-srr24.pdf 29AUG2023: 9:44

Table 2.0302: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Platelet Count < 100X10E9/L | 8 | 6 | 14 |
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 4 ( 50.0%) | 0 | 4 ( 28.6%) |
| 95% Exact CI | 0.1570, 0.8430 | 0.0000, 0.4593 | 0.0839, 0.5810 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.50 (0.11, 0.89) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.50 (0.07, 0.93) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.50 (-0.04, 0.88) | | |
| Non-Responder, n(%) | 4 ( 50.0%) | 6 (100.0%) | 10 ( 71.4%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 0 | 1 ( 16.7%) | 1 ( 7.1%) |
| >0% Spleen Volume increase at Week 24 | 1 ( 12.5%) | 0 | 1 ( 7.1%) |
| <35% Spleen Volume reduction at Week 24 | 4 ( 50.0%) | 5 ( 83.3%) | 9 ( 64.3%) |
| Last participation date < Day 141 in DB Phase | 0 | 0 | 0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol

Data Extracted: CRF data: 01JUL2019

Table 2.0302: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 2 ( 25.0%) | 0 | 2 ( 14.3%) |
| 95% Exact CI | 0.0319, 0.6509 | 0.0000, 0.4593 | 0.0178, 0.4281 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.25 (-0.11, 0.61) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.25 (-0.15, 0.65) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.25 (-0.30, 0.71) | | |
| Non-Responder, n(%) | 6 ( 75.0%) | 6 (100.0%) | 12 ( 85.7%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 12 not available | 0 | 0 | 0 |
| >0% Spleen Volume increase at Week 12 | 1 ( 12.5%) | 0 | 1 ( 7.1%) |
| <35% Spleen Volume reduction at Week 12 | 6 ( 75.0%) | 6 (100.0%) | 12 ( 85.7%) |
| Last participation date < Day 57 in DB Phase | 0 | 0 | 0 |
| Splenic Response Rate at Any Timepoint in DB Phase | | | |
| Responder, n(%) | 4 ( 50.0%) | 0 | 4 ( 28.6%) |
| 95% Exact CI | 0.1570, 0.8430 | 0.0000, 0.4593 | 0.0839, 0.5810 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol

Data Extracted: CRF data: 01JUL2019

Table 2.0302: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 15 | 16 | 31 |
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 5 ( 33.3%) | 4 ( 25.0%) | 9 ( 29.0%) |
| 95% Exact CI | 0.1182, 0.6162 | 0.0727, 0.5238 | 0.1422, 0.4804 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.18 (-0.10, 0.47) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.08 (-0.25, 0.42) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.08 (-0.28, 0.41) | | |
| Non-Responder, n(%) | 10 ( 66.7%) | 12 ( 75.0%) | 22 ( 71.0%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 1 ( 6.7%) | 1 ( 6.3%) | 2 ( 6.5%) |
| >0% Spleen Volume increase at Week 24 | 2 ( 13.3%) | 3 ( 18.8%) | 5 ( 16.1%) |
| <35% Spleen Volume reduction at Week 24 | 9 ( 60.0%) | 11 ( 68.8%) | 20 ( 64.5%) |
| Last participation date < Day 141 in DB Phase | 0 | 0 | 0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol

Data Extracted: CRF data: 01JUL2019
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Table 2.0302: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 5 ( 33.3%) | 0 | 5 ( 16.1%) |
| 95% Exact CI | 0.1182, 0.6162 | 0.0000, 0.2059 | 0.0545, 0.3373 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.33 (0.08, 0.59) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.33 (0.07, 0.60) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.33 (-0.03, 0.62) | | |
| Non-Responder, n(%) | 10 ( 66.7%) | 16 (100.0%) | 26 ( 83.9%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 12 not available | 2 ( 13.3%) | 1 ( 6.3%) | 3 ( 9.7%) |
| >0% Spleen Volume increase at Week 12 | 0 | 3 ( 18.8%) | 3 ( 9.7%) |
| <35% Spleen Volume reduction at Week 12 | 8 ( 53.3%) | 15 ( 93.8%) | 23 ( 74.2%) |
| Last participation date < Day 57 in DB Phase | 0 | 0 | 0 |
| Splenic Response Rate at Any Timepoint in DB Phase | | | |
| Responder, n(%) | 6 ( 40.0%) | 4 ( 25.0%) | 10 ( 32.3%) |
| 95% Exact CI | 0.1634, 0.6771 | 0.0727, 0.5238 | 0.1668, 0.5137 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol

Data Extracted: CRF data: 01JUL2019
Source: t-srr24-sen.sas V.03.05 Output file: t-sen-ppt-srr24.pdf 29AUG2023: 9:44

Table 2.0302: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence Yes and Platelet Count > 200X10E9/L | 14 | 14 | 28 |
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 2 ( 14.3%) | 8 ( 57.1%) | 10 ( 35.7%) |
| 95% Exact CI | 0.0178, 0.4281 | 0.2886, 0.8234 | 0.1864, 0.5593 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | -0.20 (-0.46, 0.06) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.43 (-0.77, -0.09) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.43 (-0.74, -0.03) | | |
| Non-Responder, n(%) | 12 ( 85.7%) | 6 ( 42.9%) | 18 ( 64.3%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 0 | 0 | 0 |
| >0% Spleen Volume increase at Week 24 | 3 ( 21.4%) | 1 ( 7.1%) | 4 ( 14.3%) |
| <35% Spleen Volume reduction at Week 24 | 12 ( 85.7%) | 6 ( 42.9%) | 18 ( 64.3%) |
| Last participation date < Day 141 in DB Phase | 0 | 0 | 0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol

Data Extracted: CRF data: 01JUL2019

Table 2.0302: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 3 ( 21.4%) | 7 ( 50.0%) | 10 ( 35.7%) |
| 95% Exact CI | 0.0466, 0.5080 | 0.2304, 0.7696 | 0.1864, 0.5593 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | -0.09 (-0.37, 0.20) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.29 (-0.64, 0.07) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.29 (-0.63, 0.12) | | |
| Non-Responder, n(%) | 11 ( 78.6%) | 7 ( 50.0%) | 18 ( 64.3%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 12 not available | 0 | 0 | 0 |
| >0% Spleen Volume increase at Week 12 | 2 ( 14.3%) | 1 ( 7.1%) | 3 ( 10.7%) |
| <35% Spleen Volume reduction at Week 12 | 11 ( 78.6%) | 7 ( 50.0%) | 18 ( 64.3%) |
| Last participation date < Day 57 in DB Phase | 0 | 0 | 0 |
| Splenic Response Rate at Any Timepoint in DB Phase | | | |
| Responder, n(%) | 3 ( 21.4%) | 9 ( 64.3%) | 12 ( 42.9%) |
| 95% Exact CI | 0.0466, 0.5080 | 0.3514, 0.8724 | 0.2446, 0.6282 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol

Data Extracted: CRF data: 01JUL2019
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Table 2.0302: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Platelet Count < 100X10E9/L | 3 | 4 | 7 |
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 1 ( 33.3%) | 0 | 1 ( 14.3%) |
| 95% Exact CI | 0.0084, 0.9057 | 0.0000, 0.6024 | 0.0036, 0.5787 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.33 (-0.37, 1.03) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.33 (-0.42, 1.09) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.33 (-0.43, 0.91) | | |
| Non-Responder, n(%) | 2 ( 66.7%) | 4 (100.0%) | 6 ( 85.7%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 0 | 1 ( 25.0%) | 1 ( 14.3%) |
| >0% Spleen Volume increase at Week 24 | 1 ( 33.3%) | 1 ( 25.0%) | 2 ( 28.6%) |
| <35% Spleen Volume reduction at Week 24 | 2 ( 66.7%) | 3 ( 75.0%) | 5 ( 71.4%) |
| Last participation date < Day 141 in DB Phase | 0 | 0 | 0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol

Data Extracted: CRF data: 01JUL2019
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Table 2.0302: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 1 ( 33.3%) | 0 | 1 ( 14.3%) |
| 95% Exact CI | 0.0084, 0.9057 | 0.0000, 0.6024 | 0.0036, 0.5787 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.33 (-0.37, 1.03) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.33 (-0.42, 1.09) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.33 (-0.43, 0.91) | | |
| Non-Responder, n(%) | 2 ( 66.7%) | 4 (100.0%) | 6 ( 85.7%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 12 not available | 0 | 0 | 0 |
| >0% Spleen Volume increase at Week 12 | 0 | 1 ( 25.0%) | 1 ( 14.3%) |
| <35% Spleen Volume reduction at Week 12 | 2 ( 66.7%) | 4 (100.0%) | 6 ( 85.7%) |
| Last participation date < Day 57 in DB Phase | 0 | 0 | 0 |
| Splenic Response Rate at Any Timepoint in DB Phase | | | |
| Responder, n(%) | 1 ( 33.3%) | 0 | 1 ( 14.3%) |
| 95% Exact CI | 0.0084, 0.9057 | 0.0000, 0.6024 | 0.0036, 0.5787 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol

Data Extracted: CRF data: 01JUL2019
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Table 2.0302: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 5 | | | |
| Transfusion Dependence No and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 14 | 16 | 30 |
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 8 ( 57.1%) | 4 ( 25.0%) | 12 ( 40.0%) |
| 95% Exact CI | 0.2886, 0.8234 | 0.0727, 0.5238 | 0.2266, 0.5940 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.42 (0.12, 0.72) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.32 (-0.03, 0.67) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.32 (-0.05, 0.63) | | |
| Non-Responder, n(%) | 6 ( 42.9%) | 12 ( 75.0%) | 18 ( 60.0%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 0 | 0 | 0 |
| >0% Spleen Volume increase at Week 24 | 1 ( 7.1%) | 0 | 1 ( 3.3%) |
| <35% Spleen Volume reduction at Week 24 | 6 ( 42.9%) | 12 ( 75.0%) | 18 ( 60.0%) |
| Last participation date < Day 141 in DB Phase | 0 | 0 | 0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol

Data Extracted: CRF data: 01JUL2019

Table 2.0302: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 6 ( 42.9%) | 5 ( 31.3%) | 11 ( 36.7%) |
| 95% Exact CI | 0.1766, 0.7114 | 0.1102, 0.5866 | 0.1993, 0.5614 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.24 (-0.06, 0.55) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.12 (-0.24, 0.47) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.12 (-0.25, 0.45) | | |
| Non-Responder, n(%) | 8 ( 57.1%) | 11 ( 68.8%) | 19 ( 63.3%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 12 not available | 0 | 0 | 0 |
| >0% Spleen Volume increase at Week 12 | 1 ( 7.1%) | 1 ( 6.3%) | 2 ( 6.7%) |
| <35% Spleen Volume reduction at Week 12 | 8 ( 57.1%) | 11 ( 68.8%) | 19 ( 63.3%) |
| Last participation date < Day 57 in DB Phase | 0 | 0 | 0 |
| Splenic Response Rate at Any Timepoint in DB Phase | | | |
| Responder, n(%) | 8 ( 57.1%) | 5 ( 31.3%) | 13 ( 43.3%) |
| 95% Exact CI | 0.2886, 0.8234 | 0.1102, 0.5866 | 0.2546, 0.6257 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol

Data Extracted: CRF data: 01JUL2019
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Table 2.0302: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 6 | | | |
| Transfusion Dependence No and Platelet Count > 200X10E9/L | 14 | 31 | 45 |
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 6 ( 42.9%) | 15 ( 48.4%) | 21 ( 46.7%) |
| 95% Exact CI | 0.1766, 0.7114 | 0.3015, 0.6694 | 0.3166, 0.6213 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.14 (-0.15, 0.43) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.06 (-0.38, 0.27) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.06 (-0.36, 0.26) | | |
| Non-Responder, n(%) | 8 ( 57.1%) | 16 ( 51.6%) | 24 ( 53.3%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 2 ( 14.3%) | 1 ( 3.2%) | 3 ( 6.7%) |
| >0% Spleen Volume increase at Week 24 | 0 | 2 ( 6.5%) | 2 ( 4.4%) |
| <35% Spleen Volume reduction at Week 24 | 6 ( 42.9%) | 15 ( 48.4%) | 21 ( 46.7%) |
| Last participation date < Day 141 in DB Phase | 0 | 0 | 0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol

Data Extracted: CRF data: 01JUL2019
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Table 2.0302: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 6 ( 42.9%) | 15 ( 48.4%) | 21 ( 46.7%) |
| 95% Exact CI | 0.1766, 0.7114 | 0.3015, 0.6694 | 0.3166, 0.6213 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.14 (-0.15, 0.43) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.06 (-0.38, 0.27) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.06 (-0.36, 0.26) | | |
| Non-Responder, n(%) | 8 ( 57.1%) | 16 ( 51.6%) | 24 ( 53.3%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 12 not available | 0 | 1 ( 3.2%) | 1 ( 2.2%) |
| >0% Spleen Volume increase at Week 12 | 1 ( 7.1%) | 2 ( 6.5%) | 3 ( 6.7%) |
| <35% Spleen Volume reduction at Week 12 | 8 ( 57.1%) | 15 ( 48.4%) | 23 ( 51.1%) |
| Last participation date < Day 57 in DB Phase | 0 | 0 | 0 |
| Splenic Response Rate at Any Timepoint in DB Phase | | | |
| Responder, n(%) | 7 ( 50.0%) | 18 ( 58.1%) | 25 ( 55.6%) |
| 95% Exact CI | 0.2304, 0.7696 | 0.3908, 0.7545 | 0.4000, 0.7036 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol

Data Extracted: CRF data: 01JUL2019
Source: t-srr24-sen.sas V.03.05 Output file: t-sen-ppt-srr24.pdf 29AUG2023: 9:44

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Double-blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| All Stratum Combined | | | |
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 25 ( 29.1%) | 27 ( 28.7%) | 52 ( 28.9%) |
| 95% Exact CI | 0.1978, 0.3986 | 0.1986, 0.3898 | 0.2239, 0.3610 |
| Noninferior Proportion Difference - Stratified CMH Method (95% CI) | 0.14 (0.02, 0.25) | | |
| p-value | 0.020 | | |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.12 (0.01, 0.23) | | |
| p-value | 0.037 | | |
| Superior Proportion Difference - Stratified CMH Method (95% CI) | 0.03 (-0.10, 0.16) | | |
| p-value | 0.68 | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.00 (-0.13, 0.14) | | |
| p-value | 0.96 | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.00 (-0.14, 0.15) | | |
| p-value | 1.00 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 0.99 (0.62, 1.56) | | |
| p-value [1] | 0.96 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 0.98 (0.52, 1.87) | | |
| p-value [1] | 0.96 | | |
| Unadjusted Absolute Risk Difference (95% CI) | 0.00 (-0.13, 0.14) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | 0.91 (0.58, 1.42) | | |
| p-value [2] | 0.67 | | |
| Non-Responder, n(%) | 61 ( 70.9%) | 67 ( 71.3%) | 128 ( 71.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019
Source: t-srr12-24-db-gba.sas V.03.05 Output file: t-srr12-24-db-gba.pdf 29AUG2023: 9:02

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Double-blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 12 not available | 11 ( 12.8%) | 5 ( 5.3%) | 16 ( 8.9%) |
| >0% Spleen Volume increase at Week 12 | 6 ( 7.0%) | 10 ( 10.6%) | 16 ( 8.9%) |
| <35% Spleen Volume reduction at Week 12 | 50 ( 58.1%) | 62 ( 66.0%) | 112 ( 62.2%) |
| Last participation date < Day 57 in DB phase | 8 ( 9.3%) | 0 | 8 ( 4.4%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Double-blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 27 ( 31.4%) | 31 ( 33.0%) | 58 ( 32.2%) |
| 95% Exact CI | 0.2181, 0.4230 | 0.2362, 0.4344 | 0.2546, 0.3958 |
| Noninferior Proportion Difference - Stratified CMH Method (95% CI) | 0.13 (0.01, 0.24) | | |
| p-value | 0.029 | | |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.12 (0.00, 0.23) | | |
| p-value | 0.047 | | |
| Superior Proportion Difference - Stratified CMH Method (95% CI) | 0.00 (-0.14, 0.14) | | |
| p-value | 0.98 | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.02 (-0.15, 0.12) | | |
| p-value | 0.82 | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.02 (-0.16, 0.13) | | |
| p-value | 0.87 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 1.05 (0.69, 1.61) | | |
| p-value [1] | 0.82 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 1.08 (0.57, 2.01) | | |
| p-value [1] | 0.82 | | |
| Unadjusted Absolute Risk Difference (95% CI) | -0.02 (-0.15, 0.12) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | 1.00 (0.65, 1.52) | | |
| p-value [2] | 0.98 | | |
| Non-Responder, n(%) | 59 ( 68.6%) | 63 ( 67.0%) | 122 ( 67.8%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 16 ( 18.6%) | 8 ( 8.5%) | 24 ( 13.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Double-blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| >0% Spleen Volume increase at Week 24 | 10 ( 11.6%) | 8 ( 8.5%) | 18 ( 10.0%) |
| <35% Spleen Volume reduction at Week 24 | 43 ( 50.0%) | 55 ( 58.5%) | 98 ( 54.4%) |
| Last participation date < Day 141 in DB phase | 12 ( 14.0%) | 4 ( 4.3%) | 16 ( 8.9%) |
| Splenic Response Rate at Any Timepoint in DB Phase | | | |
| Responder, n(%) | 31 ( 36.0%) | 36 ( 38.3%) | 67 ( 37.2%) |
| 95% Exact CI | 0.2597, 0.4712 | 0.2846, 0.4890 | 0.3015, 0.4473 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Double-blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Platelet Count < 100X10E9/L | 10 | 9 | 19 |
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 3 ( 30.0%) | 0 | 3 ( 15.8%) |
| 95% Exact CI | 0.0667, 0.6525 | 0.0000, 0.3363 | 0.0338, 0.3958 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.30 (-0.02, 0.62) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.30 (-0.04, 0.64) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.30 (-0.13, 0.68) | | |
| Non-Responder, n(%) | 7 ( 70.0%) | 9 (100.0%) | 16 ( 84.2%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 12 not available | 1 ( 10.0%) | 1 ( 11.1%) | 2 ( 10.5%) |
| >0% Spleen Volume increase at Week 12 | 1 ( 10.0%) | 2 ( 22.2%) | 3 ( 15.8%) |
| <35% Spleen Volume reduction at Week 12 | 6 ( 60.0%) | 8 ( 88.9%) | 14 ( 73.7%) |
| Last participation date < Day 57 in DB phase | 1 ( 10.0%) | 0 | 1 ( 5.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

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Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Double-blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 5 ( 50.0%) | 0 | 5 ( 26.3%) |
| 95% Exact CI | 0.1871, 0.8129 | 0.0000, 0.3363 | 0.0915, 0.5120 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.50 (0.16, 0.84) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.50 (0.14, 0.86) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.50 (0.08, 0.82) | | |
| Non-Responder, n(%) | 5 ( 50.0%) | 9 (100.0%) | 14 ( 73.7%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 1 ( 10.0%) | 3 ( 33.3%) | 4 ( 21.1%) |
| >0% Spleen Volume increase at Week 24 | 1 ( 10.0%) | 1 ( 11.1%) | 2 ( 10.5%) |
| <35% Spleen Volume reduction at Week 24 | 4 ( 40.0%) | 6 ( 66.7%) | 10 ( 52.6%) |
| Last participation date < Day 141 in DB phase | 1 ( 10.0%) | 2 ( 22.2%) | 3 ( 15.8%) |
| Splenic Response Rate at Any Timepoint in DB Phase | | | |
| Responder, n(%) | 5 ( 50.0%) | 0 | 5 ( 26.3%) |
| 95% Exact CI | 0.1871, 0.8129 | 0.0000, 0.3363 | 0.0915, 0.5120 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Double-blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 20 | 18 | 38 |
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 5 ( 25.0%) | 0 | 5 ( 13.2%) |
| 95% Exact CI | 0.0866, 0.4910 | 0.0000, 0.1853 | 0.0441, 0.2809 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.25 (0.05, 0.45) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.25 (0.04, 0.46) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.25 (-0.08, 0.54) | | |
| Non-Responder, n(%) | 15 ( 75.0%) | 18 (100.0%) | 33 ( 86.8%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 12 not available | 5 ( 25.0%) | 3 ( 16.7%) | 8 ( 21.1%) |
| >0% Spleen Volume increase at Week 12 | 1 ( 5.0%) | 3 ( 16.7%) | 4 ( 10.5%) |
| <35% Spleen Volume reduction at Week 12 | 10 ( 50.0%) | 15 ( 83.3%) | 25 ( 65.8%) |
| Last participation date < Day 57 in DB phase | 3 ( 15.0%) | 0 | 3 ( 7.9%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Double-blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 5 ( 25.0%) | 4 ( 22.2%) | 9 ( 23.7%) |
| 95% Exact CI | 0.0866, 0.4910 | 0.0641, 0.4764 | 0.1144, 0.4024 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.12 (-0.12, 0.35) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.03 (-0.25, 0.31) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.03 (-0.29, 0.34) | | |
| Non-Responder, n(%) | 15 ( 75.0%) | 14 ( 77.8%) | 29 ( 76.3%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 5 ( 25.0%) | 3 ( 16.7%) | 8 ( 21.1%) |
| >0% Spleen Volume increase at Week 24 | 3 ( 15.0%) | 3 ( 16.7%) | 6 ( 15.8%) |
| <35% Spleen Volume reduction at Week 24 | 10 ( 50.0%) | 11 ( 61.1%) | 21 ( 55.3%) |
| Last participation date < Day 141 in DB phase | 4 ( 20.0%) | 2 ( 11.1%) | 6 ( 15.8%) |
| Splenic Response Rate at Any Timepoint in DB Phase | | | |
| Responder, n(%) | 6 ( 30.0%) | 4 ( 22.2%) | 10 ( 26.3%) |
| 95% Exact CI | 0.1189, 0.5428 | 0.0641, 0.4764 | 0.1340, 0.4310 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Double-blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence Yes and Platelet Count > 200X10E9/L | 19 | 16 | 35 |
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 3 ( 15.8%) | 7 ( 43.8%) | 10 ( 28.6%) |
| 95% Exact CI | 0.0338, 0.3958 | 0.1975, 0.7012 | 0.1464, 0.4630 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | -0.10 (-0.34, 0.13) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.28 (-0.59, 0.03) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.28 (-0.57, 0.05) | | |
| Non-Responder, n(%) | 16 ( 84.2%) | 9 ( 56.3%) | 25 ( 71.4%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 12 not available | 2 ( 10.5%) | 0 | 2 ( 5.7%) |
| >0% Spleen Volume increase at Week 12 | 2 ( 10.5%) | 1 ( 6.3%) | 3 ( 8.6%) |
| <35% Spleen Volume reduction at Week 12 | 14 ( 73.7%) | 9 ( 56.3%) | 23 ( 65.7%) |
| Last participation date < Day 57 in DB phase | 1 ( 5.3%) | 0 | 1 ( 2.9%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

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Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Double-blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 2 ( 10.5%) | 8 ( 50.0%) | 10 ( 28.6%) |
| 95% Exact CI | 0.0130, 0.3314 | 0.2465, 0.7535 | 0.1464, 0.4630 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | -0.19 (-0.41, 0.02) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.39 (-0.69, -0.10) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.39 (-0.66, -0.06) | | |
| Non-Responder, n(%) | 17 ( 89.5%) | 8 ( 50.0%) | 25 ( 71.4%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 4 ( 21.1%) | 0 | 4 ( 11.4%) |
| >0% Spleen Volume increase at Week 24 | 3 ( 15.8%) | 1 ( 6.3%) | 4 ( 11.4%) |
| <35% Spleen Volume reduction at Week 24 | 13 ( 68.4%) | 8 ( 50.0%) | 21 ( 60.0%) |
| Last participation date < Day 141 in DB phase | 3 ( 15.8%) | 0 | 3 ( 8.6%) |
| Splenic Response Rate at Any Timepoint in DB Phase | | | |
| Responder, n(%) | 3 ( 15.8%) | 9 ( 56.3%) | 12 ( 34.3%) |
| 95% Exact CI | 0.0338, 0.3958 | 0.2988, 0.8025 | 0.1913, 0.5221 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

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Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Double-blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Platelet Count < 100X10E9/L | 3 | 4 | 7 |
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 1 ( 33.3%) | 0 | 1 ( 14.3%) |
| 95% Exact CI | 0.0084, 0.9057 | 0.0000, 0.6024 | 0.0036, 0.5787 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.33 (-0.37, 1.03) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.33 (-0.42, 1.09) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.33 (-0.43, 0.91) | | |
| Non-Responder, n(%) | 2 ( 66.7%) | 4 (100.0%) | 6 ( 85.7%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 12 not available | 0 | 0 | 0 |
| >0% Spleen Volume increase at Week 12 | 0 | 1 ( 25.0%) | 1 ( 14.3%) |
| <35% Spleen Volume reduction at Week 12 | 2 ( 66.7%) | 4 (100.0%) | 6 ( 85.7%) |
| Last participation date < Day 57 in DB phase | 0 | 0 | 0 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Double-blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 1 ( 33.3%) | 0 | 1 ( 14.3%) |
| 95% Exact CI | 0.0084, 0.9057 | 0.0000, 0.6024 | 0.0036, 0.5787 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.33 (-0.37, 1.03) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.33 (-0.42, 1.09) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.33 (-0.43, 0.91) | | |
| Non-Responder, n(%) | 2 ( 66.7%) | 4 (100.0%) | 6 ( 85.7%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 0 | 1 ( 25.0%) | 1 ( 14.3%) |
| >0% Spleen Volume increase at Week 24 | 1 ( 33.3%) | 1 ( 25.0%) | 2 ( 28.6%) |
| <35% Spleen Volume reduction at Week 24 | 2 ( 66.7%) | 3 ( 75.0%) | 5 ( 71.4%) |
| Last participation date < Day 141 in DB phase | 0 | 0 | 0 |
| Splenic Response Rate at Any Timepoint in DB Phase | | | |
| Responder, n(%) | 1 ( 33.3%) | 0 | 1 ( 14.3%) |
| 95% Exact CI | 0.0084, 0.9057 | 0.0000, 0.6024 | 0.0036, 0.5787 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Double-blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 5 | | | |
| Transfusion Dependence No and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 16 | 16 | 32 |
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 7 ( 43.8%) | 5 ( 31.3%) | 12 ( 37.5%) |
| 95% Exact CI | 0.1975, 0.7012 | 0.1102, 0.5866 | 0.2110, 0.5631 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.25 (-0.04, 0.54) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.13 (-0.22, 0.47) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.13 (-0.25, 0.47) | | |
| Non-Responder, n(%) | 9 ( 56.3%) | 11 ( 68.8%) | 20 ( 62.5%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 12 not available | 1 ( 6.3%) | 0 | 1 ( 3.1%) |
| >0% Spleen Volume increase at Week 12 | 1 ( 6.3%) | 1 ( 6.3%) | 2 ( 6.3%) |
| <35% Spleen Volume reduction at Week 12 | 8 ( 50.0%) | 11 ( 68.8%) | 19 ( 59.4%) |
| Last participation date < Day 57 in DB phase | 1 ( 6.3%) | 0 | 1 ( 3.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

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Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Double-blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 8 ( 50.0%) | 4 ( 25.0%) | 12 ( 37.5%) |
| 95% Exact CI | 0.2465, 0.7535 | 0.0727, 0.5238 | 0.2110, 0.5631 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.35 (0.06, 0.64) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.25 (-0.09, 0.59) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.25 (-0.13, 0.58) | | |
| Non-Responder, n(%) | 8 ( 50.0%) | 12 ( 75.0%) | 20 ( 62.5%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 1 ( 6.3%) | 0 | 1 ( 3.1%) |
| >0% Spleen Volume increase at Week 24 | 1 ( 6.3%) | 0 | 1 ( 3.1%) |
| <35% Spleen Volume reduction at Week 24 | 7 ( 43.8%) | 12 ( 75.0%) | 19 ( 59.4%) |
| Last participation date < Day 141 in DB phase | 2 ( 12.5%) | 0 | 2 ( 6.3%) |
| Splenic Response Rate at Any Timepoint in DB Phase | | | |
| Responder, n(%) | 9 ( 56.3%) | 5 ( 31.3%) | 14 ( 43.8%) |
| 95% Exact CI | 0.2988, 0.8025 | 0.1102, 0.5866 | 0.2636, 0.6234 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

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Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Double-blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 6 | | | |
| Transfusion Dependence No and Platelet Count > 200X10E9/L | 18 | 31 | 49 |
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 6 ( 33.3%) | 15 ( 48.4%) | 21 ( 42.9%) |
| 95% Exact CI | 0.1334, 0.5901 | 0.3015, 0.6694 | 0.2882, 0.5779 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.04 (-0.21, 0.29) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.15 (-0.44, 0.14) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.15 (-0.42, 0.14) | | |
| Non-Responder, n(%) | 12 ( 66.7%) | 16 ( 51.6%) | 28 ( 57.1%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 12 not available | 2 ( 11.1%) | 1 ( 3.2%) | 3 ( 6.1%) |
| >0% Spleen Volume increase at Week 12 | 1 ( 5.6%) | 2 ( 6.5%) | 3 ( 6.1%) |
| <35% Spleen Volume reduction at Week 12 | 10 ( 55.6%) | 15 ( 48.4%) | 25 ( 51.0%) |
| Last participation date < Day 57 in DB phase | 2 ( 11.1%) | 0 | 2 ( 4.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019

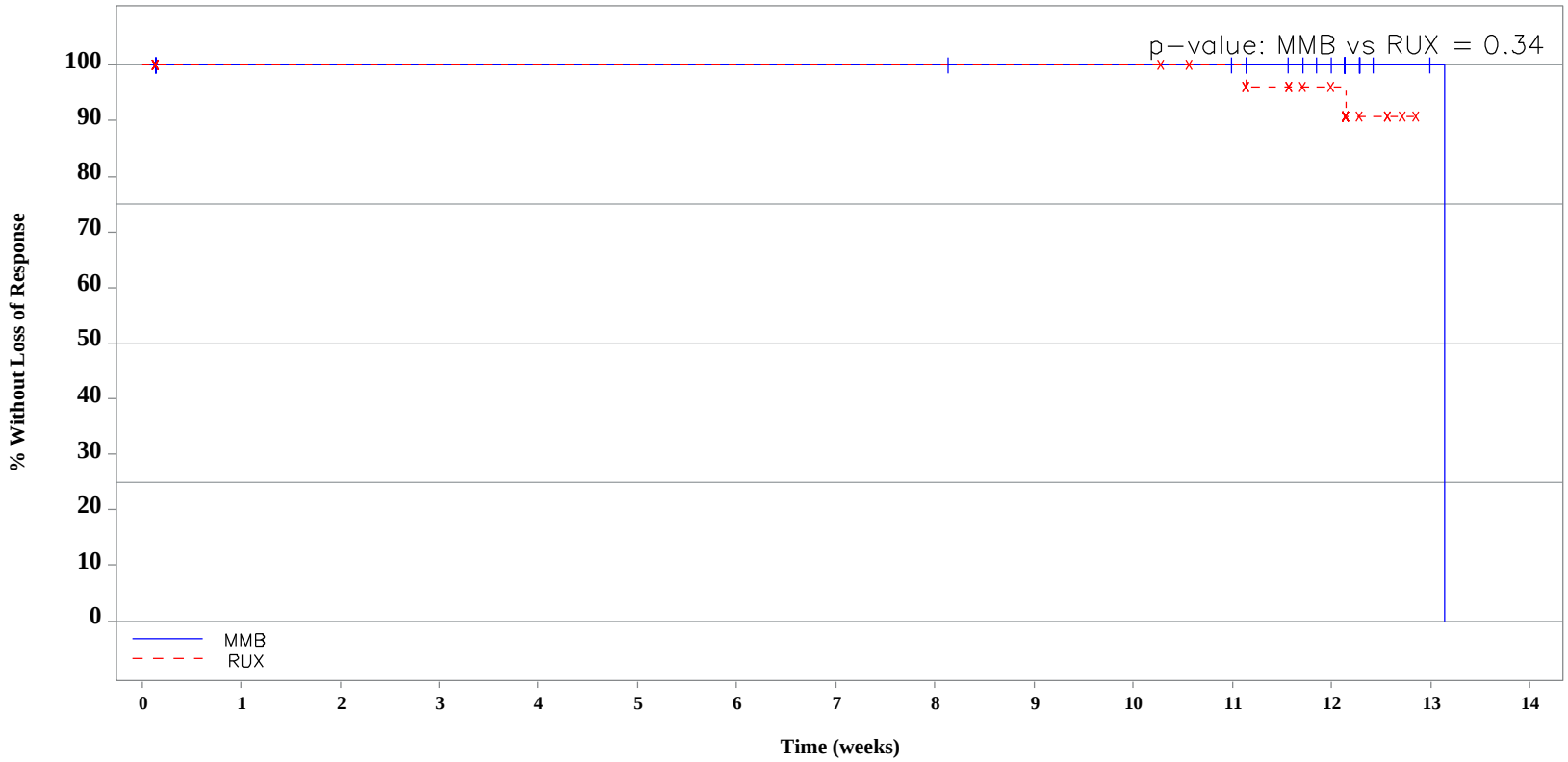
Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Double-blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 6 ( 33.3%) | 15 ( 48.4%) | 21 ( 42.9%) |
| 95% Exact CI | 0.1334, 0.5901 | 0.3015, 0.6694 | 0.2882, 0.5779 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.04 (-0.21, 0.29) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.15 (-0.44, 0.14) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.15 (-0.42, 0.14) | | |
| Non-Responder, n(%) | 12 ( 66.7%) | 16 ( 51.6%) | 28 ( 57.1%) |
| Baseline Spleen Volume not available | 0 | 0 | 0 |
| Spleen Volume at Week 24 not available | 5 ( 27.8%) | 1 ( 3.2%) | 6 ( 12.2%) |
| >0% Spleen Volume increase at Week 24 | 1 ( 5.6%) | 2 ( 6.5%) | 3 ( 6.1%) |
| <35% Spleen Volume reduction at Week 24 | 7 ( 38.9%) | 15 ( 48.4%) | 22 ( 44.9%) |
| Last participation date < Day 141 in DB phase | 2 ( 11.1%) | 0 | 2 ( 4.1%) |
| Splenic Response Rate at Any Timepoint in DB Phase | | | |
| Responder, n(%) | 7 ( 38.9%) | 18 ( 58.1%) | 25 ( 51.0%) |
| 95% Exact CI | 0.1730, 0.6425 | 0.3908, 0.7545 | 0.3634, 0.6558 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019
Source: t-srr12-24-db-gba.sas V.03.05 Output file: t-srr12-24-db-gba.pdf 29AUG2023: 9:02

Figure 2.0601: Kaplan-Meier Plot of Duration of MRI/CT Splenic Response
Double-blind Phase
ITT-Anemic Analysis Set with >= 35% Spleen Volume Reduction from Baseline in DB Phase




**N at Risk (Events)**

| | | | | | | | | | | | | | | | |
|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|
| **MMB** | 31 (0) | 26 (0) | 26 (0) | 26 (0) | 26 (0) | 26 (0) | 26 (0) | 26 (0) | 26 (0) | 25 (0) | 25 (0) | 25 (0) | 19 (0) | 3 (0) | 0 (2) |
| **RUX** | 36 (0) | 27 (0) | 27 (0) | 27 (0) | 27 (0) | 27 (0) | 27 (0) | 27 (0) | 27 (0) | 27 (0) | 27 (0) | 25 (0) | 19 (1) | 0 (2) | 0 (2) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
Stratified two-sided p-value is from a log-rank test comparing treatment stratified by baseline TD (yes,no) and baseline platelet count (<100,100-200,>200 10^9/L).

Data Extracted: CRF data: 01JUL2019
Source: g-dur-sr.sas V.03.05 Output file: g-dur-sr35.pdf 17OCT2023:19:54

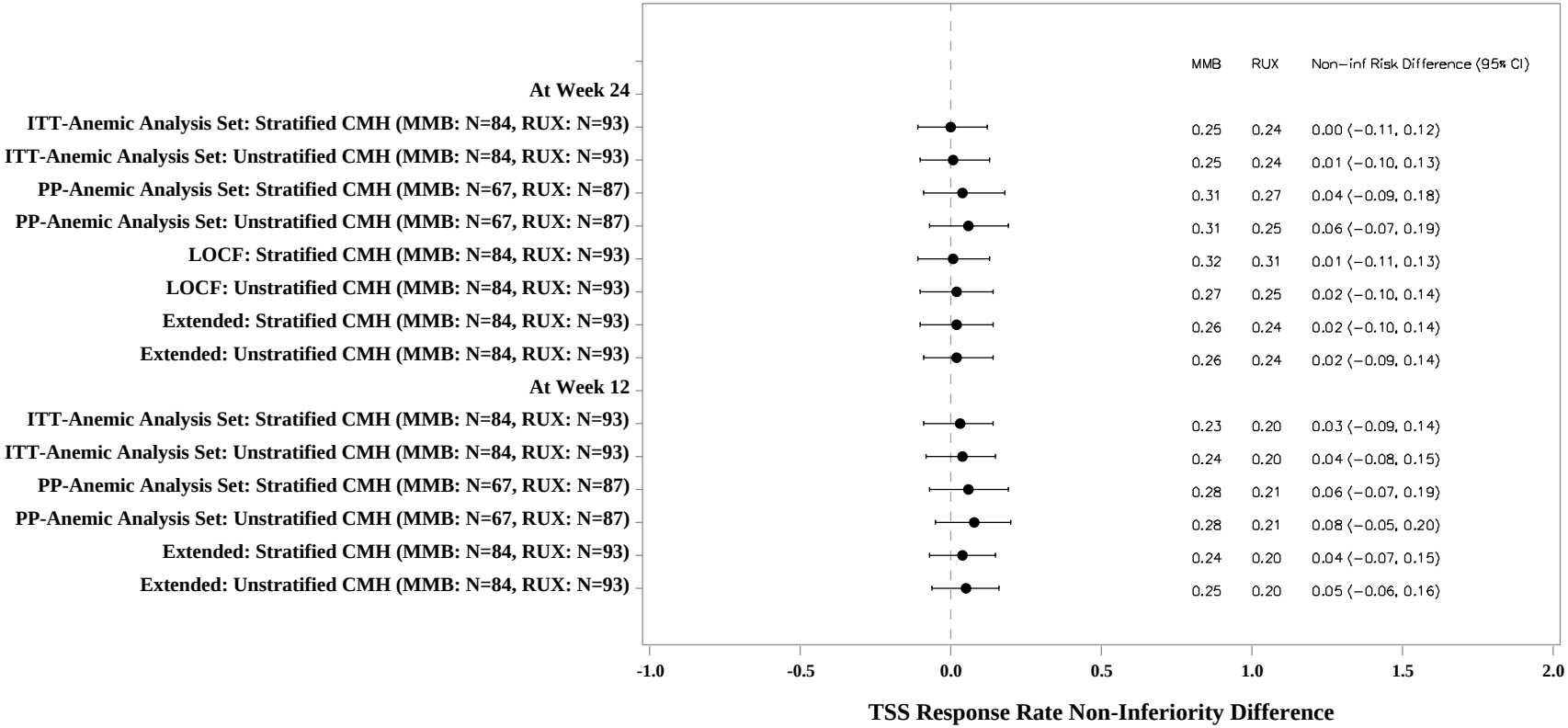
Table 2.0501: Analysis of Duration of MRI/CT Splenic Response
Double-blind Phase
ITT-Anemic Analysis Set with >= 35% Spleen Volume Reduction from Baseline in DB Phase

| | MMB (N=31) | RUX (N=36) |
|---|---|---|
| **Subjects with Event** | | |
| Loss of spleen response, n(%) | 2 ( 6.5%) | 2 ( 5.6%) |
| **Censor** | | |
| Subjects Censored, n(%) | 29 ( 93.5%) | 34 ( 94.4%) |
| Non-Responder: Censored at Randomization Date | 0 | 0 |
| No Loss of Response: Censored at Last MRI Spleen Assessment Date in DB phase (including up to 10 days after First Dose Date of OL phase) | 29 (100.0%) | 34 (100.0%) |
| **Kaplan-Meier Estimate of Duration of Spleen Response (Weeks)** | | |
| 25-percentile (95% CI) | 13.14 (NE, NE) | NE (11.14, NE) |
| Median (95% CI) | 13.14 (NE, NE) | NE (NE, NE) |
| 75-percentile (95% CI) | 13.14 (NE, NE) | NE (NE, NE) |
| Min, Max | 0.14, 13.14 | 0.14, 12.86 |
| Stratified Log-Rank Test p-value | 0.34 | |
| Adjusted Hazard Ratio (95% CI) | <0.01 (<0.01, NE) | |
| Unstratified Log-Rank Test p-value | 0.16 | |
| Unadjusted Hazard Ratio (95% CI) | <0.01 (<0.01, NE) | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
Stratified two-sided p-value is from a log-rank test comparing treatment stratified by baseline TD (yes,no) and baseline platelet count (<100,100-200,>200 10^9/L). Adjusted hazard ratio and 95% CI are from a Cox proportional hazards model with covariates of treatment, baseline TD (yes,no) and baseline platelet count (<100,100-200,>200 10^9/L).
Unstratified two-sided p-value is from a log-rank test comparing treatment.
Unadjusted hazard ratio and 95% CI are from a Cox proportional hazards model with a covariate of treatment.
MRI = Magnetic Resonance Imaging. CT = Computerized Tomography.

Data Extracted: CRF data: 01JUL2019
Source: g-dur-sr.sas V.03.05 Output file: t-dur-sr35.pdf 17OCT2023:19:53

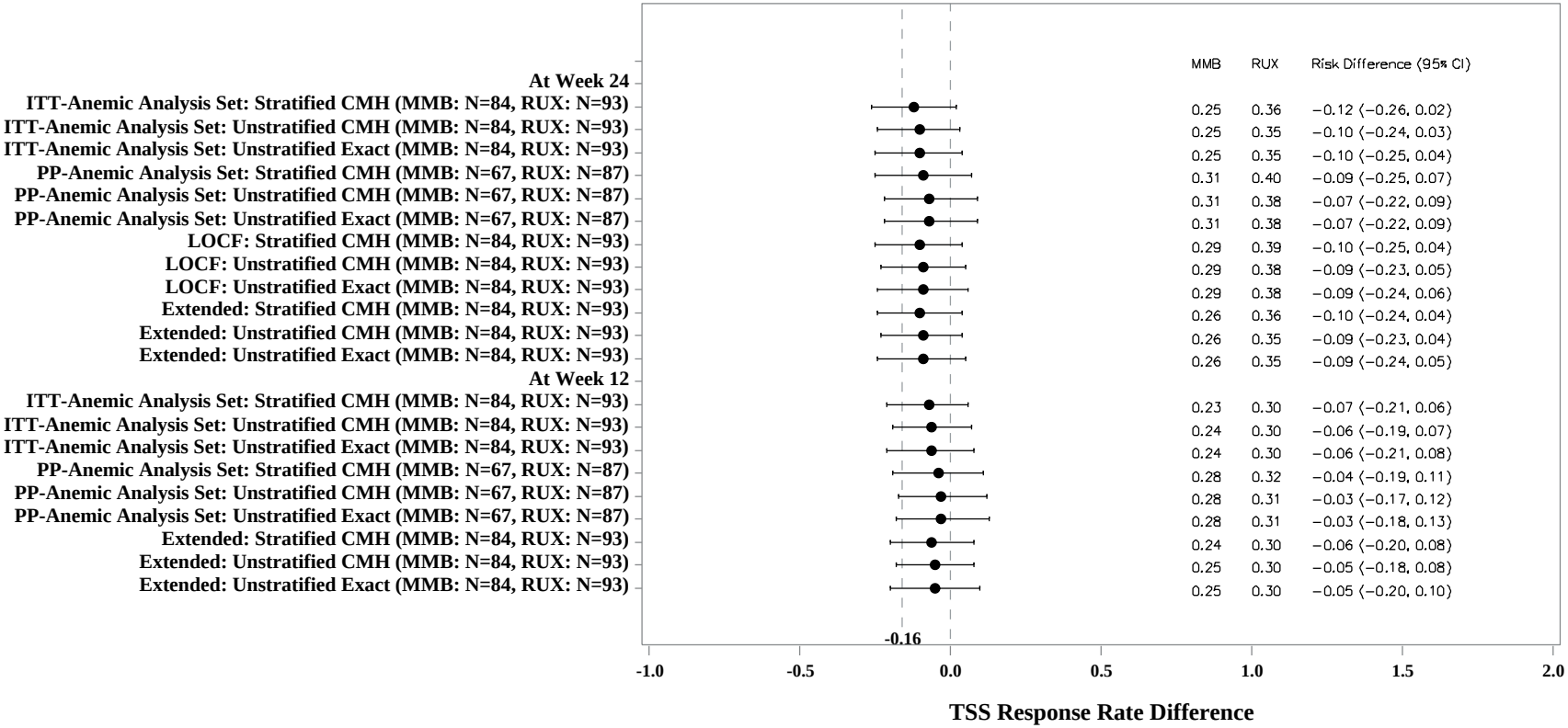
Figure 2.1001: Forest plot of Primary and Sensitivity Analysis of Response Rate in TSS at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic and PP-Anemic Analysis Sets
Noninferiority




ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
To the right of the reference line favors MMB, to the left favors RUX.
Non-inferiority risk difference calculated on a factor of 0.67.
CMH = Cochran-Mantel-Haenszel; LOCF = Last Observation Carried Forward; PP=Per-Protocol; CI= Confidence Interval
TSS = Total Symptom Score

Data Extracted: CRF data: 01JUL2019
Source: g-tss-forest.sas V.03.05 Output file: g-tss-forest.pdf 30AUG2023:12:28

Figure 2.1001: Forest plot of Primary and Sensitivity Analysis of Response Rate in TSS at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic and PP-Anemic Analysis Sets
Superiority and Fixed Margin Noninferiority




ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
To the right of the reference line favors MMB, to the left favors RUX.
Non-inferiority risk difference calculated on a factor of 0.67.
CMH = Cochran-Mantel-Haenszel; LOCF = Last Observation Carried Forward; PP=Per-Protocol; CI= Confidence Interval
TSS = Total Symptom Score

Data Extracted: CRF data: 01JUL2019
Source: g-tss-forest.sas V.03.05 Output file: g-tss-forest.pdf 30AUG2023:12:28

Table 2.1302: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| All Strata Combined | | | |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 1 ( 1.2%) | 1 ( 1.1%) | 2 ( 1.1%) |
| TSS = 0 at baseline | 2 ( 2.3%) | 0 | 2 ( 1.1%) |
| TSS > 0 at baseline | 83 ( 96.5%) | 93 ( 98.9%) | 176 ( 97.8%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 84 | 93 | 177 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 1 ( 1.2%) | 0 | 1 ( 0.6%) |
| Responder, n(%) | 22 ( 26.2%) | 33 ( 35.5%) | 55 ( 31.1%) |
| 95% Exact CI | 0.1720, 0.3693 | 0.2583, 0.4609 | 0.2434, 0.3845 |
| Noninferior Proportion Difference - Stratified CMH Method (95% CI) | 0.02 (-0.10, 0.14) | | |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.02 (-0.09, 0.14) | | |
| Superior Proportion Difference - Stratified CMH Method (95% CI) | -0.10 (-0.24, 0.04) | | |
| p-value | 0.16 | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.09 (-0.23, 0.04) | | |
| p-value | 0.18 | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.09 (-0.24, 0.05) | | |
| p-value | 0.20 | | |
| Non-Responder, n(%) | 62 ( 73.8%) | 60 ( 64.5%) | 122 ( 68.9%) |
| Last participation date < Day 162 in DB phase | 16 ( 19.0%) | 8 ( 8.6%) | 24 ( 13.6%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 2 ( 2.4%) | 3 ( 3.2%) | 5 ( 2.8%) |
| >0% increase from baseline at Week 24 | 17 ( 20.2%) | 14 ( 15.1%) | 31 ( 17.5%) |
| <50% reduction from baseline Week 24 | 43 ( 51.2%) | 49 ( 52.7%) | 92 ( 52.0%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92).Day 1 is defined as the date of randomization.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
Source: t-tss24-sen.sas V.03.05 Output file: t-sen-ext-tss24.pdf 29AUG2023: 9:47

Table 2.1302: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 84 | 93 | 177 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 1 ( 1.2%) | 0 | 1 ( 0.6%) |
| Responder, n(%) | 21 ( 25.0%) | 28 ( 30.1%) | 49 ( 27.7%) |
| 95% Exact CI | 0.1619, 0.3564 | 0.2103, 0.4050 | 0.2124, 0.3490 |
| Noninferior Proportion Difference - Stratified CMH Method (95% CI) | 0.04 (-0.07, 0.15) | | |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.05 (-0.06, 0.16) | | |
| Superior Proportion Difference - Stratified CMH Method (95% CI) | -0.06 (-0.20, 0.08) | | |
| p-value | 0.38 | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.05 (-0.18, 0.08) | | |
| p-value | 0.45 | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.05 (-0.20, 0.10) | | |
| p-value | 0.50 | | |
| Non-Responder, n(%) | 63 ( 75.0%) | 65 ( 69.9%) | 128 ( 72.3%) |
| Last participation date < Day 78 in DB phase | 10 ( 11.9%) | 0 | 10 ( 5.6%) |
| Last participation date >= Day 78 and TSS at Week 12 not available | 1 ( 1.2%) | 1 ( 1.1%) | 2 ( 1.1%) |
| >0% increase from baseline at Week 12 | 21 ( 25.0%) | 19 ( 20.4%) | 40 ( 22.6%) |
| <50% reduction from baseline at Week 12 | 51 ( 60.7%) | 64 ( 68.8%) | 115 ( 65.0%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92).Day 1 is defined as the date of randomization.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
Source: t-tss24-sen.sas V.03.05 Output file: t-sen-ext-tss24.pdf 29AUG2023: 9:47

Table 2.1302: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Platelet Count < 100X10E9/L | 10 | 9 | 19 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 1 ( 10.0%) | 0 | 1 ( 5.3%) |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 9 ( 90.0%) | 9 (100.0%) | 18 ( 94.7%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 9 | 9 | 18 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 2 ( 22.2%) | 3 ( 33.3%) | 5 ( 27.8%) |
| 95% Exact CI | 0.0281, 0.6001 | 0.0749, 0.7007 | 0.0969, 0.5348 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.00 (-0.37, 0.37) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.11 (-0.56, 0.34) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.11 (-0.57, 0.38) | | |
| Non-Responder, n(%) | 7 ( 77.8%) | 6 ( 66.7%) | 13 ( 72.2%) |
| Last participation date < Day 162 in DB phase | 1 ( 11.1%) | 3 ( 33.3%) | 4 ( 22.2%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 0 | 1 ( 11.1%) | 1 ( 5.6%) |
| >0% increase from baseline at Week 24 | 4 ( 44.4%) | 0 | 4 ( 22.2%) |
| <50% reduction from baseline Week 24 | 6 ( 66.7%) | 2 ( 22.2%) | 8 ( 44.4%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92).Day 1 is defined as the date of randomization.
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
Source: t-tss24-sen.sas V.03.05  Output file: t-sen-ext-tss24.pdf 29AUG2023: 9:47

Table 2.1302: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 9 | 9 | 18 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 0 | 0 |
| Responder, n(%) | 2 ( 22.2%) | 3 ( 33.3%) | 5 ( 27.8%) |
| 95% Exact CI | 0.0281, 0.6001 | 0.0749, 0.7007 | 0.0969, 0.5348 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.00 (-0.37, 0.37) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.11 (-0.56, 0.34) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.11 (-0.57, 0.38) | | |
| Non-Responder, n(%) | 7 ( 77.8%) | 6 ( 66.7%) | 13 ( 72.2%) |
| Last participation date < Day 78 in DB phase | 1 ( 11.1%) | 0 | 1 ( 5.6%) |
| Last participation date >= Day 78 and TSS at Week 12 not available | 0 | 1 ( 11.1%) | 1 ( 5.6%) |
| >0% increase from baseline at Week 12 | 5 ( 55.6%) | 1 ( 11.1%) | 6 ( 33.3%) |
| <50% reduction from baseline at Week 12 | 6 ( 66.7%) | 5 ( 55.6%) | 11 ( 61.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92).Day 1 is defined as the date of randomization.
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019

Table 2.1302: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 20 | 18 | 38 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 1 ( 5.0%) | 0 | 1 ( 2.6%) |
| TSS > 0 at baseline | 19 ( 95.0%) | 18 (100.0%) | 37 ( 97.4%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 19 | 18 | 37 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 6 ( 31.6%) | 7 ( 38.9%) | 13 ( 35.1%) |
| 95% Exact CI | 0.1258, 0.5655 | 0.1730, 0.6425 | 0.2021, 0.5254 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.06 (-0.21, 0.32) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.07 (-0.39, 0.24) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.07 (-0.38, 0.26) | | |
| Non-Responder, n(%) | 13 ( 68.4%) | 11 ( 61.1%) | 24 ( 64.9%) |
| Last participation date < Day 162 in DB phase | 5 ( 26.3%) | 4 ( 22.2%) | 9 ( 24.3%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 1 ( 5.3%) | 0 | 1 ( 2.7%) |
| >0% increase from baseline at Week 24 | 3 ( 15.8%) | 3 ( 16.7%) | 6 ( 16.2%) |
| <50% reduction from baseline Week 24 | 7 ( 36.8%) | 7 ( 38.9%) | 14 ( 37.8%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92).Day 1 is defined as the date of randomization.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
Source: t-tss24-sen.sas V.03.05 Output file: t-sen-ext-tss24.pdf 29AUG2023: 9:47

Table 2.1302: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 19 | 18 | 37 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 0 | 0 |
| Responder, n(%) | 7 ( 36.8%) | 7 ( 38.9%) | 14 ( 37.8%) |
| 95% Exact CI | 0.1629, 0.6164 | 0.1730, 0.6425 | 0.2246, 0.5524 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.11 (-0.16, 0.38) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.02 (-0.34, 0.30) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.02 (-0.33, 0.31) | | |
| Non-Responder, n(%) | 12 ( 63.2%) | 11 ( 61.1%) | 23 ( 62.2%) |
| Last participation date < Day 78 in DB phase | 3 ( 15.8%) | 0 | 3 ( 8.1%) |
| Last participation date >= Day 78 and TSS at Week 12 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 12 | 2 ( 10.5%) | 3 ( 16.7%) | 5 ( 13.5%) |
| <50% reduction from baseline at Week 12 | 9 ( 47.4%) | 11 ( 61.1%) | 20 ( 54.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92).Day 1 is defined as the date of randomization.
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
Source: t-tss24-sen.sas V.03.05  Output file: t-sen-ext-tss24.pdf 29AUG2023: 9:47

Table 2.1302: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence Yes and Platelet Count > 200X10E9/L | 19 | 16 | 35 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 1 ( 6.3%) | 1 ( 2.9%) |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 19 (100.0%) | 15 ( 93.8%) | 34 ( 97.1%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 19 | 15 | 34 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 4 ( 21.1%) | 6 ( 40.0%) | 10 ( 29.4%) |
| 95% Exact CI | 0.0605, 0.4557 | 0.1634, 0.6771 | 0.1510, 0.4748 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | -0.06 (-0.32, 0.20) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.19 (-0.51, 0.13) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.19 (-0.50, 0.15) | | |
| Non-Responder, n(%) | 15 ( 78.9%) | 9 ( 60.0%) | 24 ( 70.6%) |
| Last participation date < Day 162 in DB phase | 3 ( 15.8%) | 0 | 3 ( 8.8%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 0 | 1 ( 6.7%) | 1 ( 2.9%) |
| >0% increase from baseline at Week 24 | 5 ( 26.3%) | 1 ( 6.7%) | 6 ( 17.6%) |
| <50% reduction from baseline Week 24 | 12 ( 63.2%) | 8 ( 53.3%) | 20 ( 58.8%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92).Day 1 is defined as the date of randomization.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
Source: t-tss24-sen.sas V.03.05 Output file: t-sen-ext-tss24.pdf 29AUG2023: 9:47

Table 2.1302: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 19 | 15 | 34 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 0 | 0 |
| Responder, n(%) | 5 ( 26.3%) | 4 ( 26.7%) | 9 ( 26.5%) |
| 95% Exact CI | 0.0915, 0.5120 | 0.0779, 0.5510 | 0.1288, 0.4436 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.08 (-0.18, 0.34) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.00 (-0.32, 0.31) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.00 (-0.33, 0.33) | | |
| Non-Responder, n(%) | 14 ( 73.7%) | 11 ( 73.3%) | 25 ( 73.5%) |
| Last participation date < Day 78 in DB phase | 3 ( 15.8%) | 0 | 3 ( 8.8%) |
| Last participation date >= Day 78 and TSS at Week 12 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 12 | 5 ( 26.3%) | 2 ( 13.3%) | 7 ( 20.6%) |
| <50% reduction from baseline at Week 12 | 11 ( 57.9%) | 11 ( 73.3%) | 22 ( 64.7%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92).Day 1 is defined as the date of randomization.
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
Source: t-tss24-sen.sas V.03.05  Output file: t-sen-ext-tss24.pdf 29AUG2023: 9:47

Table 2.1302: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Platelet Count < 100X10E9/L | 3 | 4 | 7 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 3 (100.0%) | 4 (100.0%) | 7 (100.0%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 3 | 4 | 7 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 2 ( 66.7%) | 2 ( 50.0%) | 4 ( 57.1%) |
| 95% Exact CI | 0.0943, 0.9916 | 0.0676, 0.9324 | 0.1841, 0.9010 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.33 (-0.44, 1.10) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.17 (-0.71, 1.04) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.17 (-0.59, 0.79) | | |
| Non-Responder, n(%) | 1 ( 33.3%) | 2 ( 50.0%) | 3 ( 42.9%) |
| Last participation date < Day 162 in DB phase | 0 | 1 ( 25.0%) | 1 ( 14.3%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 24 | 1 ( 33.3%) | 0 | 1 ( 14.3%) |
| <50% reduction from baseline Week 24 | 1 ( 33.3%) | 1 ( 25.0%) | 2 ( 28.6%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92).Day 1 is defined as the date of randomization.
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019

Table 2.1302: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 3 | 4 | 7 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 0 | 0 |
| Responder, n(%) | 2 ( 66.7%) | 2 ( 50.0%) | 4 ( 57.1%) |
| 95% Exact CI | 0.0943, 0.9916 | 0.0676, 0.9324 | 0.1841, 0.9010 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.33 (-0.44, 1.10) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.17 (-0.71, 1.04) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.17 (-0.59, 0.79) | | |
| Non-Responder, n(%) | 1 ( 33.3%) | 2 ( 50.0%) | 3 ( 42.9%) |
| Last participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Last participation date >= Day 78 and TSS at Week 12 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 12 | 1 ( 33.3%) | 1 ( 25.0%) | 2 ( 28.6%) |
| <50% reduction from baseline at Week 12 | 1 ( 33.3%) | 2 ( 50.0%) | 3 ( 42.9%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92).Day 1 is defined as the date of randomization.
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019

Table 2.1302: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 5 | | | |
| Transfusion Dependence No and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 16 | 16 | 32 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 16 (100.0%) | 16 (100.0%) | 32 (100.0%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 16 | 16 | 32 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 4 ( 25.0%) | 7 ( 43.8%) | 11 ( 34.4%) |
| 95% Exact CI | 0.0727, 0.5238 | 0.1975, 0.7012 | 0.1857, 0.5319 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | -0.04 (-0.32, 0.24) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.19 (-0.52, 0.15) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.19 (-0.53, 0.19) | | |
| Non-Responder, n(%) | 12 ( 75.0%) | 9 ( 56.3%) | 21 ( 65.6%) |
| Last participation date < Day 162 in DB phase | 2 ( 12.5%) | 0 | 2 ( 6.3%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 24 | 2 ( 12.5%) | 3 ( 18.8%) | 5 ( 15.6%) |
| <50% reduction from baseline Week 24 | 10 ( 62.5%) | 9 ( 56.3%) | 19 ( 59.4%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92).Day 1 is defined as the date of randomization.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019

Table 2.1302: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 16 | 16 | 32 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 0 | 0 |
| Responder, n(%) | 3 ( 18.8%) | 4 ( 25.0%) | 7 ( 21.9%) |
| 95% Exact CI | 0.0405, 0.4565 | 0.0727, 0.5238 | 0.0928, 0.3997 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.02 (-0.23, 0.27) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.06 (-0.37, 0.24) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.06 (-0.42, 0.31) | | |
| Non-Responder, n(%) | 13 ( 81.3%) | 12 ( 75.0%) | 25 ( 78.1%) |
| Last participation date < Day 78 in DB phase | 1 ( 6.3%) | 0 | 1 ( 3.1%) |
| Last participation date >= Day 78 and TSS at Week 12 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 12 | 4 ( 25.0%) | 5 ( 31.3%) | 9 ( 28.1%) |
| <50% reduction from baseline at Week 12 | 12 ( 75.0%) | 12 ( 75.0%) | 24 ( 75.0%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92).Day 1 is defined as the date of randomization.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.1302: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 6 | | | |
| Transfusion Dependence No and Platelet Count > 200X10E9/L | 18 | 31 | 49 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 1 ( 5.6%) | 0 | 1 ( 2.0%) |
| TSS > 0 at baseline | 17 ( 94.4%) | 31 (100.0%) | 48 ( 98.0%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 18 | 31 | 49 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 1 ( 5.6%) | 0 | 1 ( 2.0%) |
| Responder, n(%) | 4 ( 22.2%) | 8 ( 25.8%) | 12 ( 24.5%) |
| 95% Exact CI | 0.0641, 0.4764 | 0.1186, 0.4461 | 0.1334, 0.3887 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.05 (-0.18, 0.28) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.04 (-0.29, 0.22) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.04 (-0.32, 0.25) | | |
| Non-Responder, n(%) | 14 ( 77.8%) | 23 ( 74.2%) | 37 ( 75.5%) |
| Last participation date < Day 162 in DB phase | 5 ( 27.8%) | 0 | 5 ( 10.2%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 1 ( 5.6%) | 1 ( 3.2%) | 2 ( 4.1%) |
| >0% increase from baseline at Week 24 | 2 ( 11.1%) | 7 ( 22.6%) | 9 ( 18.4%) |
| <50% reduction from baseline Week 24 | 7 ( 38.9%) | 22 ( 71.0%) | 29 ( 59.2%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92).Day 1 is defined as the date of randomization.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.1302: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 18 | 31 | 49 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 1 ( 5.6%) | 0 | 1 ( 2.0%) |
| Responder, n(%) | 2 ( 11.1%) | 8 ( 25.8%) | 10 ( 20.4%) |
| 95% Exact CI | 0.0138, 0.3471 | 0.1186, 0.4461 | 0.1024, 0.3434 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | -0.06 (-0.25, 0.13) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.15 (-0.37, 0.08) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.15 (-0.42, 0.14) | | |
| Non-Responder, n(%) | 16 ( 88.9%) | 23 ( 74.2%) | 39 ( 79.6%) |
| Last participation date < Day 78 in DB phase | 2 ( 11.1%) | 0 | 2 ( 4.1%) |
| Last participation date >= Day 78 and TSS at Week 12 not available | 1 ( 5.6%) | 0 | 1 ( 2.0%) |
| >0% increase from baseline at Week 12 | 4 ( 22.2%) | 7 ( 22.6%) | 11 ( 22.4%) |
| <50% reduction from baseline at Week 12 | 12 ( 66.7%) | 23 ( 74.2%) | 35 ( 71.4%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92).Day 1 is defined as the date of randomization.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.1301: Sensitivity Analysis of Response Rate in TSS at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| All Strata Combined | | | |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 1 ( 1.2%) | 1 ( 1.1%) | 2 ( 1.1%) |
| TSS = 0 at baseline | 2 ( 2.3%) | 0 | 2 ( 1.1%) |
| TSS > 0 at baseline | 83 ( 96.5%) | 93 ( 98.9%) | 176 ( 97.8%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 84 | 93 | 177 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 1 ( 1.2%) | 0 | 1 ( 0.6%) |
| Responder at Week 24, n(%) | 21 ( 25.0%) | 33 ( 35.5%) | 54 ( 30.5%) |
| Responder from LOCF, n(%) | 3 ( 3.6%) | 2 ( 2.2%) | 5 ( 2.8%) |
| Noninferior Proportion Difference - Stratified CMH Method (95% CI) | 0.01 (-0.11, 0.13) | | |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.02 (-0.10, 0.14) | | |
| Superior Proportion Difference - Stratified CMH Method (95% CI) | -0.10 (-0.25, 0.04) | | |
| p-value | 0.16 | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.09 (-0.23, 0.05) | | |
| p-value | 0.20 | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.09 (-0.24, 0.06) | | |
| p-value | 0.26 | | |
| Non-Responder, n(%) | 60 ( 71.4%) | 58 ( 62.4%) | 118 ( 66.7%) |
| Last participation date < Day 162 in DB phase | 16 ( 19.0%) | 8 ( 8.6%) | 24 ( 13.6%) |
| TSS at Week 24 not available | 8 ( 9.5%) | 0 | 8 ( 4.5%) |
| >0% increase from baseline at Week 24 | 21 ( 25.0%) | 18 ( 19.4%) | 39 ( 22.0%) |
| <50% reduction from baseline Week 24 | 51 ( 60.7%) | 58 ( 62.4%) | 109 ( 61.6%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
LOCF = Last Observation Carried Forward
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.1301: Sensitivity Analysis of Response Rate in TSS at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Platelet Count < 100X10E9/L | 10 | 9 | 19 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 1 ( 10.0%) | 0 | 1 ( 5.3%) |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 9 ( 90.0%) | 9 (100.0%) | 18 ( 94.7%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 9 | 9 | 18 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder at Week 24, n(%) | 2 ( 22.2%) | 3 ( 33.3%) | 5 ( 27.8%) |
| Responder from LOCF, n(%) | 0 | 0 | 0 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.00 (-0.37, 0.37) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.11 (-0.56, 0.34) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.11 (-0.57, 0.38) | | |
| Non-Responder, n(%) | 7 ( 77.8%) | 6 ( 66.7%) | 13 ( 72.2%) |
| Last participation date < Day 162 in DB phase | 1 ( 11.1%) | 3 ( 33.3%) | 4 ( 22.2%) |
| TSS at Week 24 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 24 | 5 ( 55.6%) | 2 ( 22.2%) | 7 ( 38.9%) |
| <50% reduction from baseline Week 24 | 7 ( 77.8%) | 6 ( 66.7%) | 13 ( 72.2%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
LOCF = Last Observation Carried Forward
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.1301: Sensitivity Analysis of Response Rate in TSS at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 20 | 18 | 38 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 1 ( 5.0%) | 0 | 1 ( 2.6%) |
| TSS > 0 at baseline | 19 ( 95.0%) | 18 (100.0%) | 37 ( 97.4%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 19 | 18 | 37 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder at Week 24, n(%) | 6 ( 31.6%) | 7 ( 38.9%) | 13 ( 35.1%) |
| Responder from LOCF, n(%) | 2 ( 10.5%) | 2 ( 11.1%) | 4 ( 10.8%) |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.05 (-0.22, 0.33) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.08 (-0.41, 0.25) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.08 (-0.40, 0.26) | | |
| Non-Responder, n(%) | 11 ( 57.9%) | 9 ( 50.0%) | 20 ( 54.1%) |
| Last participation date < Day 162 in DB phase | 5 ( 26.3%) | 4 ( 22.2%) | 9 ( 24.3%) |
| TSS at Week 24 not available | 1 ( 5.3%) | 0 | 1 ( 2.7%) |
| >0% increase from baseline at Week 24 | 4 ( 21.1%) | 4 ( 22.2%) | 8 ( 21.6%) |
| <50% reduction from baseline Week 24 | 10 ( 52.6%) | 9 ( 50.0%) | 19 ( 51.4%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
LOCF = Last Observation Carried Forward
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.1301: Sensitivity Analysis of Response Rate in TSS at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence Yes and Platelet Count > 200X10E9/L | 19 | 16 | 35 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 1 ( 6.3%) | 1 ( 2.9%) |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 19 (100.0%) | 15 ( 93.8%) | 34 ( 97.1%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 19 | 15 | 34 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder at Week 24, n(%) | 4 ( 21.1%) | 6 ( 40.0%) | 10 ( 29.4%) |
| Responder from LOCF, n(%) | 0 | 0 | 0 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | -0.06 (-0.32, 0.20) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.19 (-0.51, 0.13) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.19 (-0.50, 0.15) | | |
| Non-Responder, n(%) | 15 ( 78.9%) | 9 ( 60.0%) | 24 ( 70.6%) |
| Last participation date < Day 162 in DB phase | 3 ( 15.8%) | 0 | 3 ( 8.8%) |
| TSS at Week 24 not available | 2 ( 10.5%) | 0 | 2 ( 5.9%) |
| >0% increase from baseline at Week 24 | 5 ( 26.3%) | 1 ( 6.7%) | 6 ( 17.6%) |
| <50% reduction from baseline Week 24 | 13 ( 68.4%) | 9 ( 60.0%) | 22 ( 64.7%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
LOCF = Last Observation Carried Forward
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
Source: t-tss24-sen.sas V.03.05 Output file: t-sen-locf-tss24.pdf 29AUG2023: 9:46

Table 2.1301: Sensitivity Analysis of Response Rate in TSS at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Platelet Count < 100X10E9/L | 3 | 4 | 7 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 3 (100.0%) | 4 (100.0%) | 7 (100.0%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 3 | 4 | 7 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder at Week 24, n(%) | 2 ( 66.7%) | 2 ( 50.0%) | 4 ( 57.1%) |
| Responder from LOCF, n(%) | 0 | 0 | 0 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.33 (-0.44, 1.10) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.17 (-0.71, 1.04) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.17 (-0.59, 0.79) | | |
| Non-Responder, n(%) | 1 ( 33.3%) | 2 ( 50.0%) | 3 ( 42.9%) |
| Last participation date < Day 162 in DB phase | 0 | 1 ( 25.0%) | 1 ( 14.3%) |
| TSS at Week 24 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 24 | 1 ( 33.3%) | 1 ( 25.0%) | 2 ( 28.6%) |
| <50% reduction from baseline Week 24 | 1 ( 33.3%) | 2 ( 50.0%) | 3 ( 42.9%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
LOCF = Last Observation Carried Forward
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
Source: t-tss24-sen.sas V.03.05  Output file: t-sen-locf-tss24.pdf 29AUG2023: 9:46

Table 2.1301: Sensitivity Analysis of Response Rate in TSS at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 5 | | | |
| Transfusion Dependence No and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 16 | 16 | 32 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 16 (100.0%) | 16 (100.0%) | 32 (100.0%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 16 | 16 | 32 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder at Week 24, n(%) | 4 ( 25.0%) | 7 ( 43.8%) | 11 ( 34.4%) |
| Responder from LOCF, n(%) | 0 | 0 | 0 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | -0.04 (-0.32, 0.24) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.19 (-0.52, 0.15) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.19 (-0.53, 0.19) | | |
| Non-Responder, n(%) | 12 ( 75.0%) | 9 ( 56.3%) | 21 ( 65.6%) |
| Last participation date < Day 162 in DB phase | 2 ( 12.5%) | 0 | 2 ( 6.3%) |
| TSS at Week 24 not available | 1 ( 6.3%) | 0 | 1 ( 3.1%) |
| >0% increase from baseline at Week 24 | 2 ( 12.5%) | 3 ( 18.8%) | 5 ( 15.6%) |
| <50% reduction from baseline Week 24 | 11 ( 68.8%) | 9 ( 56.3%) | 20 ( 62.5%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
LOCF = Last Observation Carried Forward
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.1301: Sensitivity Analysis of Response Rate in TSS at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 6 | | | |
| Transfusion Dependence No and Platelet Count > 200X10E9/L | 18 | 31 | 49 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 1 ( 5.6%) | 0 | 1 ( 2.0%) |
| TSS > 0 at baseline | 17 ( 94.4%) | 31 (100.0%) | 48 ( 98.0%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 18 | 31 | 49 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 1 ( 5.6%) | 0 | 1 ( 2.0%) |
| Responder at Week 24, n(%) | 3 ( 16.7%) | 8 ( 25.8%) | 11 ( 22.4%) |
| Responder from LOCF, n(%) | 1 ( 5.6%) | 0 | 1 ( 2.0%) |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.03 (-0.19, 0.25) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.04 (-0.29, 0.22) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.04 (-0.32, 0.25) | | |
| Non-Responder, n(%) | 14 ( 77.8%) | 23 ( 74.2%) | 37 ( 75.5%) |
| Last participation date < Day 162 in DB phase | 5 ( 27.8%) | 0 | 5 ( 10.2%) |
| TSS at Week 24 not available | 4 ( 22.2%) | 0 | 4 ( 8.2%) |
| >0% increase from baseline at Week 24 | 4 ( 22.2%) | 7 ( 22.6%) | 11 ( 22.4%) |
| <50% reduction from baseline Week 24 | 9 ( 50.0%) | 23 ( 74.2%) | 32 ( 65.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
LOCF = Last Observation Carried Forward
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
Source: t-tss24-sen.sas V.03.05 Output file: t-sen-locf-tss24.pdf 29AUG2023: 9:46

Table 2.1303: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| All Strata Combined | | | |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 1 ( 1.5%) | 0 | 1 ( 0.6%) |
| TSS > 0 at baseline | 67 ( 98.5%) | 87 (100.0%) | 154 ( 99.4%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 67 | 87 | 154 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 21 ( 31.3%) | 33 ( 37.9%) | 54 ( 35.1%) |
| 95% Exact CI | 0.2056, 0.4384 | 0.2774, 0.4897 | 0.2756, 0.4316 |
| Noninferior Proportion Difference - Stratified CMH Method (95% CI) | 0.04 (-0.09, 0.18) | | |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.06 (-0.07, 0.19) | | |
| Superior Proportion Difference - Stratified CMH Method (95% CI) | -0.09 (-0.25, 0.07) | | |
| p-value | 0.28 | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.07 (-0.22, 0.09) | | |
| p-value | 0.40 | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.07 (-0.22, 0.09) | | |
| p-value | 0.50 | | |
| Non-Responder, n(%) | 46 ( 68.7%) | 54 ( 62.1%) | 100 ( 64.9%) |
| Last participation date < Day 162 in DB phase | 2 ( 3.0%) | 4 ( 4.6%) | 6 ( 3.9%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 3 ( 4.5%) | 4 ( 4.6%) | 7 ( 4.5%) |
| >0% increase from baseline at Week 24 | 16 ( 23.9%) | 13 ( 14.9%) | 29 ( 18.8%) |
| <50% reduction from baseline Week 24 | 41 ( 61.2%) | 46 ( 52.9%) | 87 ( 56.5%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.1303: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 67 | 87 | 154 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 0 | 0 |
| Responder, n(%) | 19 ( 28.4%) | 27 ( 31.0%) | 46 ( 29.9%) |
| 95% Exact CI | 0.1801, 0.4069 | 0.2155, 0.4186 | 0.2277, 0.3776 |
| Noninferior Proportion Difference - Stratified CMH Method (95% CI) | 0.06 (-0.07, 0.19) | | |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.08 (-0.05, 0.20) | | |
| Superior Proportion Difference - Stratified CMH Method (95% CI) | -0.04 (-0.19, 0.11) | | |
| p-value | 0.59 | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.03 (-0.17, 0.12) | | |
| p-value | 0.72 | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.03 (-0.18, 0.13) | | |
| p-value | 0.86 | | |
| Non-Responder, n(%) | 48 ( 71.6%) | 60 ( 69.0%) | 108 ( 70.1%) |
| Last participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Last participation date >= Day 78 and TSS at Week 12 not available | 2 ( 3.0%) | 2 ( 2.3%) | 4 ( 2.6%) |
| >0% increase from baseline at Week 12 | 18 ( 26.9%) | 17 ( 19.5%) | 35 ( 22.7%) |
| <50% reduction from baseline at Week 12 | 46 ( 68.7%) | 58 ( 66.7%) | 104 ( 67.5%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.1303: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Platelet Count < 100X10E9/L | 8 | 6 | 14 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 8 (100.0%) | 6 (100.0%) | 14 (100.0%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 8 | 6 | 14 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 2 ( 25.0%) | 3 ( 50.0%) | 5 ( 35.7%) |
| 95% Exact CI | 0.0319, 0.6509 | 0.1181, 0.8819 | 0.1276, 0.6486 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | -0.09 (-0.53, 0.36) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.25 (-0.80, 0.30) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.25 (-0.71, 0.30) | | |
| Non-Responder, n(%) | 6 ( 75.0%) | 3 ( 50.0%) | 9 ( 64.3%) |
| Last participation date < Day 162 in DB phase | 0 | 1 ( 16.7%) | 1 ( 7.1%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 24 | 4 ( 50.0%) | 0 | 4 ( 28.6%) |
| <50% reduction from baseline Week 24 | 6 ( 75.0%) | 2 ( 33.3%) | 8 ( 57.1%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.1303: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 8 | 6 | 14 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 0 | 0 |
| Responder, n(%) | 2 ( 25.0%) | 3 ( 50.0%) | 5 ( 35.7%) |
| 95% Exact CI | 0.0319, 0.6509 | 0.1181, 0.8819 | 0.1276, 0.6486 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | -0.09 (-0.53, 0.36) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.25 (-0.80, 0.30) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.25 (-0.71, 0.30) | | |
| Non-Responder, n(%) | 6 ( 75.0%) | 3 ( 50.0%) | 9 ( 64.3%) |
| Last participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Last participation date >= Day 78 and TSS at Week 12 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 12 | 5 ( 62.5%) | 1 ( 16.7%) | 6 ( 42.9%) |
| <50% reduction from baseline at Week 12 | 6 ( 75.0%) | 3 ( 50.0%) | 9 ( 64.3%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019

Table 2.1303: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 15 | 16 | 31 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 1 ( 6.7%) | 0 | 1 ( 3.2%) |
| TSS > 0 at baseline | 14 ( 93.3%) | 16 (100.0%) | 30 ( 96.8%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 14 | 16 | 30 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 6 ( 42.9%) | 7 ( 43.8%) | 13 ( 43.3%) |
| 95% Exact CI | 0.1766, 0.7114 | 0.1975, 0.7012 | 0.2546, 0.6257 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.14 (-0.18, 0.45) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.01 (-0.38, 0.36) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.01 (-0.37, 0.35) | | |
| Non-Responder, n(%) | 8 ( 57.1%) | 9 ( 56.3%) | 17 ( 56.7%) |
| Last participation date < Day 162 in DB phase | 0 | 2 ( 12.5%) | 2 ( 6.7%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 1 ( 7.1%) | 0 | 1 ( 3.3%) |
| >0% increase from baseline at Week 24 | 3 ( 21.4%) | 3 ( 18.8%) | 6 ( 20.0%) |
| <50% reduction from baseline Week 24 | 7 ( 50.0%) | 7 ( 43.8%) | 14 ( 46.7%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.1303: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 14 | 16 | 30 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 0 | 0 |
| Responder, n(%) | 6 ( 42.9%) | 6 ( 37.5%) | 12 ( 40.0%) |
| 95% Exact CI | 0.1766, 0.7114 | 0.1520, 0.6457 | 0.2266, 0.5940 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.18 (-0.14, 0.49) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.05 (-0.31, 0.42) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.05 (-0.31, 0.40) | | |
| Non-Responder, n(%) | 8 ( 57.1%) | 10 ( 62.5%) | 18 ( 60.0%) |
| Last participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Last participation date >= Day 78 and TSS at Week 12 not available | 1 ( 7.1%) | 0 | 1 ( 3.3%) |
| >0% increase from baseline at Week 12 | 1 ( 7.1%) | 2 ( 12.5%) | 3 ( 10.0%) |
| <50% reduction from baseline at Week 12 | 7 ( 50.0%) | 10 ( 62.5%) | 17 ( 56.7%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.1303: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence Yes and Platelet Count > 200X10E9/L | 14 | 14 | 28 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 14 (100.0%) | 14 (100.0%) | 28 (100.0%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 14 | 14 | 28 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 4 ( 28.6%) | 6 ( 42.9%) | 10 ( 35.7%) |
| 95% Exact CI | 0.0839, 0.5810 | 0.1766, 0.7114 | 0.1864, 0.5593 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.00 (-0.31, 0.31) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.14 (-0.51, 0.22) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.14 (-0.51, 0.26) | | |
| Non-Responder, n(%) | 10 ( 71.4%) | 8 ( 57.1%) | 18 ( 64.3%) |
| Last participation date < Day 162 in DB phase | 0 | 0 | 0 |
| Last participation date >= Day 162 and TSS at Week 24 not available | 0 | 1 (  7.1%) | 1 (  3.6%) |
| >0% increase from baseline at Week 24 | 4 ( 28.6%) | 1 (  7.1%) | 5 ( 17.9%) |
| <50% reduction from baseline Week 24 | 10 ( 71.4%) | 7 ( 50.0%) | 17 ( 60.7%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.1303: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 14 | 14 | 28 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 0 | 0 |
| Responder, n(%) | 4 ( 28.6%) | 4 ( 28.6%) | 8 ( 28.6%) |
| 95% Exact CI | 0.0839, 0.5810 | 0.0839, 0.5810 | 0.1322, 0.4867 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.09 (-0.21, 0.39) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.00 (-0.35, 0.35) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.00 (-0.39, 0.39) | | |
| Non-Responder, n(%) | 10 ( 71.4%) | 10 ( 71.4%) | 20 ( 71.4%) |
| Last participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Last participation date >= Day 78 and TSS at Week 12 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 12 | 4 ( 28.6%) | 2 ( 14.3%) | 6 ( 21.4%) |
| <50% reduction from baseline at Week 12 | 10 ( 71.4%) | 10 ( 71.4%) | 20 ( 71.4%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.1303: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Platelet Count < 100X10E9/L | 3 | 4 | 7 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 3 (100.0%) | 4 (100.0%) | 7 (100.0%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 3 | 4 | 7 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 2 ( 66.7%) | 2 ( 50.0%) | 4 ( 57.1%) |
| 95% Exact CI | 0.0943, 0.9916 | 0.0676, 0.9324 | 0.1841, 0.9010 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.33 (-0.44, 1.10) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.17 (-0.71, 1.04) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.17 (-0.59, 0.79) | | |
| Non-Responder, n(%) | 1 ( 33.3%) | 2 ( 50.0%) | 3 ( 42.9%) |
| Last participation date < Day 162 in DB phase | 0 | 1 ( 25.0%) | 1 ( 14.3%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 24 | 1 ( 33.3%) | 0 | 1 ( 14.3%) |
| <50% reduction from baseline Week 24 | 1 ( 33.3%) | 1 ( 25.0%) | 2 ( 28.6%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.1303: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 3 | 4 | 7 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 0 | 0 |
| Responder, n(%) | 2 ( 66.7%) | 2 ( 50.0%) | 4 ( 57.1%) |
| 95% Exact CI | 0.0943, 0.9916 | 0.0676, 0.9324 | 0.1841, 0.9010 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.33 (-0.44, 1.10) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | 0.17 (-0.71, 1.04) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | 0.17 (-0.59, 0.79) | | |
| Non-Responder, n(%) | 1 ( 33.3%) | 2 ( 50.0%) | 3 ( 42.9%) |
| Last participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Last participation date >= Day 78 and TSS at Week 12 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 12 | 1 ( 33.3%) | 1 ( 25.0%) | 2 ( 28.6%) |
| <50% reduction from baseline at Week 12 | 1 ( 33.3%) | 2 ( 50.0%) | 3 ( 42.9%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.1303: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 5 | | | |
| Transfusion Dependence No and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 14 | 16 | 30 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 14 (100.0%) | 16 (100.0%) | 30 (100.0%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 14 | 16 | 30 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 4 ( 28.6%) | 7 ( 43.8%) | 11 ( 36.7%) |
| 95% Exact CI | 0.0839, 0.5810 | 0.1975, 0.7012 | 0.1993, 0.5614 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | -0.01 (-0.31, 0.29) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.15 (-0.51, 0.20) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.15 (-0.49, 0.21) | | |
| Non-Responder, n(%) | 10 ( 71.4%) | 9 ( 56.3%) | 19 ( 63.3%) |
| Last participation date < Day 162 in DB phase | 0 | 0 | 0 |
| Last participation date >= Day 162 and TSS at Week 24 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 24 | 2 ( 14.3%) | 3 ( 18.8%) | 5 ( 16.7%) |
| <50% reduction from baseline Week 24 | 10 ( 71.4%) | 9 ( 56.3%) | 19 ( 63.3%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.1303: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 14 | 16 | 30 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 0 | 0 |
| Responder, n(%) | 3 ( 21.4%) | 4 ( 25.0%) | 7 ( 23.3%) |
| 95% Exact CI | 0.0466, 0.5080 | 0.0727, 0.5238 | 0.0993, 0.4228 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.05 (-0.23, 0.32) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.04 (-0.36, 0.28) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.04 (-0.38, 0.32) | | |
| Non-Responder, n(%) | 11 ( 78.6%) | 12 ( 75.0%) | 23 ( 76.7%) |
| Last participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Last participation date >= Day 78 and TSS at Week 12 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 12 | 4 ( 28.6%) | 5 ( 31.3%) | 9 ( 30.0%) |
| <50% reduction from baseline at Week 12 | 11 ( 78.6%) | 12 ( 75.0%) | 23 ( 76.7%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019

Table 2.1303: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 6 | | | |
| Transfusion Dependence No and Platelet Count > 200X10E9/L | 14 | 31 | 45 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 14 (100.0%) | 31 (100.0%) | 45 (100.0%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 14 | 31 | 45 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 3 ( 21.4%) | 8 ( 25.8%) | 11 ( 24.4%) |
| 95% Exact CI | 0.0466, 0.5080 | 0.1186, 0.4461 | 0.1288, 0.3954 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | 0.04 (-0.21, 0.29) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.04 (-0.32, 0.24) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.04 (-0.35, 0.27) | | |
| Non-Responder, n(%) | 11 ( 78.6%) | 23 ( 74.2%) | 34 ( 75.6%) |
| Last participation date < Day 162 in DB phase | 2 ( 14.3%) | 0 | 2 ( 4.4%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 2 ( 14.3%) | 3 ( 9.7%) | 5 ( 11.1%) |
| >0% increase from baseline at Week 24 | 2 ( 14.3%) | 6 ( 19.4%) | 8 ( 17.8%) |
| <50% reduction from baseline Week 24 | 7 ( 50.0%) | 20 ( 64.5%) | 27 ( 60.0%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.1303: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 14 | 31 | 45 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 0 | 0 |
| Responder, n(%) | 2 ( 14.3%) | 8 ( 25.8%) | 10 ( 22.2%) |
| 95% Exact CI | 0.0178, 0.4281 | 0.1186, 0.4461 | 0.1120, 0.3709 |
| Noninferior Proportion Difference - Unstratified CMH Method (95% CI) | -0.03 (-0.26, 0.20) | | |
| Superior Proportion Difference - Unstratified CMH Method (95% CI) | -0.12 (-0.37, 0.14) | | |
| Superior Proportion Difference - Unstratified Exact Method (95% CI) | -0.12 (-0.42, 0.20) | | |
| Non-Responder, n(%) | 12 ( 85.7%) | 23 ( 74.2%) | 35 ( 77.8%) |
| Last participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Last participation date >= Day 78 and TSS at Week 12 not available | 1 ( 7.1%) | 2 ( 6.5%) | 3 ( 6.7%) |
| >0% increase from baseline at Week 12 | 3 ( 21.4%) | 6 ( 19.4%) | 9 ( 20.0%) |
| <50% reduction from baseline at Week 12 | 11 ( 78.6%) | 21 ( 67.7%) | 32 ( 71.1%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 01JUL2019
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Table 2.0701: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 1 ( 1.2%) | 1 ( 1.1%) | 2 ( 1.1%) |
| TSS = 0 at baseline | 2 ( 2.3%) | 0 | 2 ( 1.1%) |
| TSS > 0 at baseline | 83 ( 96.5%) | 93 ( 98.9%) | 176 ( 97.8%) |
| Response Rate of Total Symptom Score at Week 4 | | | |
| Subjects Evaluable at Week 4, n | 85 | 93 | 178 |
| TSS = 0 at baseline and TSS >0 or missing at Week 4 | 2 ( 2.4%) | 0 | 2 ( 1.1%) |
| Responder, n(%) | 6 ( 7.1%) | 17 ( 18.3%) | 23 ( 12.9%) |
| 95% Exact CI | 0.0263, 0.1473 | 0.1102, 0.2765 | 0.0837, 0.1876 |
| Proportion Difference - Stratified CMH Method (95% CI) | -0.12 (-0.23, -0.01) | | |
| p-value | 0.035 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | -0.11 (-0.21, -0.01) | | |
| p-value | 0.024 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | -0.11 (-0.26, 0.03) | | |
| p-value | 0.042 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 2.59 (1.07, 6.26) | | |
| p-value [1] | 0.035 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 2.95 (1.10, 7.87) | | |
| p-value [1] | 0.031 | | |
| Unadjusted Absolute Risk Difference (95% CI) | -0.11 (-0.21, -0.02) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | NE | | |
| p-value [2] | NE | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
Data Extracted: CRF data: 01JUL2019
Source: t-tss24-overall.sas V.03.05 Output file: t-tss24-gba-db.pdf 29AUG2023: 9:54

Table 2.0701: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Non-Responder, n(%) | 79 ( 92.9%) | 76 ( 81.7%) | 155 ( 87.1%) |
| Last participation date < Day 21 in DB Phase | 3 ( 3.5%) | 0 | 3 ( 1.7%) |
| Last participation date >= Day 21 and TSS at Week 4 not available | 4 ( 4.7%) | 2 ( 2.2%) | 6 ( 3.4%) |
| >0% increase from baseline at Week 4 | 24 ( 28.2%) | 15 ( 16.1%) | 39 ( 21.9%) |
| <50% reduction from baseline at Week 4 | 70 ( 82.4%) | 74 ( 79.6%) | 144 ( 80.9%) |
| Return Rate (%) | 92% | 98% | 95% |
| Number of Subjects in Risk | 83 | 94 | 177 |
| Return Rate in Risk (%) | 95% | 98% | 97% |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
Data Extracted: CRF data: 01JUL2019
Source: t-tss24-overall.sas V.03.05 Output file: t-tss24-gba-db.pdf 29AUG2023: 9:54

Table 2.0701: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 8 | | | |
| Subjects Evaluable at Week 8, n | 85 | 93 | 178 |
| TSS = 0 at baseline and TSS >0 or missing at Week 8 | 2 ( 2.4%) | 0 | 2 ( 1.1%) |
| Responder, n(%) | 16 ( 18.8%) | 24 ( 25.8%) | 40 ( 22.5%) |
| 95% Exact CI | 0.1116, 0.2876 | 0.1729, 0.3592 | 0.1657, 0.2932 |
| Proportion Difference - Stratified CMH Method (95% CI) | -0.08 (-0.21, 0.05) | | |
| p-value | 0.22 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | -0.07 (-0.19, 0.05) | | |
| p-value | 0.27 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | -0.07 (-0.21, 0.08) | | |
| p-value | 0.29 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 1.37 (0.78, 2.40) | | |
| p-value [1] | 0.27 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 1.50 (0.73, 3.07) | | |
| p-value [1] | 0.27 | | |
| Unadjusted Absolute Risk Difference (95% CI) | -0.07 (-0.19, 0.05) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | 1.44 (0.82, 2.51) | | |
| p-value [2] | 0.20 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
Data Extracted: CRF data: 01JUL2019

Table 2.0701: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Non-Responder, n(%) | 69 ( 81.2%) | 69 ( 74.2%) | 138 ( 77.5%) |
| Last participation date < Day 50 in DB Phase | 7 ( 8.2%) | 0 | 7 ( 3.9%) |
| Last participation date >= Day 50 and TSS at Week 8 not available | 3 ( 3.5%) | 2 ( 2.2%) | 5 ( 2.8%) |
| >0% increase from baseline at Week 8 | 18 ( 21.2%) | 16 ( 17.2%) | 34 ( 19.1%) |
| <50% reduction from baseline at Week 8 | 57 ( 67.1%) | 67 ( 72.0%) | 124 ( 69.7%) |
| Return Rate (%) | 88% | 98% | 93% |
| Number of Subjects in Risk | 79 | 94 | 173 |
| Return Rate in Risk (%) | 96% | 98% | 97% |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
Data Extracted: CRF data: 01JUL2019
Source: t-tss24-overall.sas V.03.05 Output file: t-tss24-gba-db.pdf 29AUG2023: 9:54

Table 2.0701: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 84 | 93 | 177 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 1 ( 1.2%) | 0 | 1 ( 0.6%) |
| Responder, n(%) | 21 ( 25.0%) | 28 ( 30.1%) | 49 ( 27.7%) |
| 95% Exact CI | 0.1619, 0.3564 | 0.2103, 0.4050 | 0.2124, 0.3490 |
| Proportion Difference - Stratified CMH Method (95% CI) | -0.06 (-0.20, 0.07) | | |
| p-value | 0.37 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | -0.05 (-0.18, 0.08) | | |
| p-value | 0.45 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | -0.05 (-0.20, 0.10) | | |
| p-value | 0.50 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 1.20 (0.74, 1.95) | | |
| p-value [1] | 0.45 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 1.29 (0.67, 2.51) | | |
| p-value [1] | 0.45 | | |
| Unadjusted Absolute Risk Difference (95% CI) | -0.05 (-0.18, 0.08) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | 1.25 (0.78, 2.01) | | |
| p-value [2] | 0.35 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
Data Extracted: CRF data: 01JUL2019

Table 2.0701: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Non-Responder, n(%) | 63 ( 75.0%) | 65 ( 69.9%) | 128 ( 72.3%) |
| Last participation date < Day 78 in DB Phase | 10 ( 11.9%) | 0 | 10 ( 5.6%) |
| Last participation date >= Day 78 and TSS at Week 12 not available | 2 ( 2.4%) | 4 ( 4.3%) | 6 ( 3.4%) |
| >0% increase from baseline at Week 12 | 17 ( 20.2%) | 17 ( 18.3%) | 34 ( 19.2%) |
| <50% reduction from baseline at Week 12 | 50 ( 59.5%) | 61 ( 65.6%) | 111 ( 62.7%) |
| Return Rate (%) | 86% | 96% | 91% |
| Number of Subjects in Risk | 76 | 94 | 170 |
| Return Rate in Risk (%) | 97% | 96% | 96% |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
Data Extracted: CRF data: 01JUL2019
Source: t-tss24-overall.sas V.03.05 Output file: t-tss24-gba-db.pdf 29AUG2023: 9:54

Table 2.0701: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 16 | | | |
| Subjects Evaluable at Week 16, n | 84 | 93 | 177 |
| TSS = 0 at baseline and TSS >0 or missing at Week 16 | 1 ( 1.2%) | 0 | 1 ( 0.6%) |
| Responder, n(%) | 21 ( 25.0%) | 28 ( 30.1%) | 49 ( 27.7%) |
| 95% Exact CI | 0.1619, 0.3564 | 0.2103, 0.4050 | 0.2124, 0.3490 |
| Proportion Difference - Stratified CMH Method (95% CI) | -0.05 (-0.19, 0.08) | | |
| p-value | 0.45 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | -0.05 (-0.18, 0.08) | | |
| p-value | 0.45 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | -0.05 (-0.20, 0.10) | | |
| p-value | 0.50 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 1.20 (0.74, 1.95) | | |
| p-value [1] | 0.45 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 1.29 (0.67, 2.51) | | |
| p-value [1] | 0.45 | | |
| Unadjusted Absolute Risk Difference (95% CI) | -0.05 (-0.18, 0.08) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | 1.22 (0.75, 1.97) | | |
| p-value [2] | 0.43 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
Data Extracted: CRF data: 01JUL2019

Table 2.0701: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Non-Responder, n(%) | 63 ( 75.0%) | 65 ( 69.9%) | 128 ( 72.3%) |
| Last participation date < Day 106 in DB Phase | 10 ( 11.9%) | 4 ( 4.3%) | 14 ( 7.9%) |
| Last participation date >= Day 106 and TSS at Week 16 not available | 2 ( 2.4%) | 4 ( 4.3%) | 6 ( 3.4%) |
| >0% increase from baseline at Week 16 | 23 ( 27.4%) | 20 ( 21.5%) | 43 ( 24.3%) |
| <50% reduction from baseline at Week 16 | 50 ( 59.5%) | 57 ( 61.3%) | 107 ( 60.5%) |
| Return Rate (%) | 86% | 91% | 89% |
| Number of Subjects in Risk | 76 | 90 | 166 |
| Return Rate in Risk (%) | 97% | 96% | 96% |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
Data Extracted: CRF data: 01JUL2019
Source: t-tss24-overall.sas V.03.05 Output file: t-tss24-gba-db.pdf 29AUG2023: 9:54

Table 2.0701: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 20 | | | |
| Subjects Evaluable at Week 20, n | 85 | 93 | 178 |
| TSS = 0 at baseline and TSS >0 or missing at Week 20 | 2 ( 2.4%) | 0 | 2 ( 1.1%) |
| Responder, n(%) | 23 ( 27.1%) | 27 ( 29.0%) | 50 ( 28.1%) |
| 95% Exact CI | 0.1799, 0.3779 | 0.2008, 0.3936 | 0.2162, 0.3530 |
| Proportion Difference - Stratified CMH Method (95% CI) | -0.03 (-0.17, 0.11) | | |
| p-value | 0.68 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | -0.02 (-0.15, 0.11) | | |
| p-value | 0.77 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | -0.02 (-0.17, 0.13) | | |
| p-value | 0.87 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 1.07 (0.67, 1.72) | | |
| p-value [1] | 0.77 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 1.10 (0.57, 2.12) | | |
| p-value [1] | 0.77 | | |
| Unadjusted Absolute Risk Difference (95% CI) | -0.02 (-0.15, 0.11) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | 1.11 (0.69, 1.79) | | |
| p-value [2] | 0.67 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
Data Extracted: CRF data: 01JUL2019

Table 2.0701: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Non-Responder, n(%) | 62 ( 72.9%) | 66 ( 71.0%) | 128 ( 71.9%) |
| Last participation date < Day 134 in DB Phase | 11 ( 12.9%) | 4 ( 4.3%) | 15 ( 8.4%) |
| Last participation date >= Day 134 and TSS at Week 20 not available | 3 ( 3.5%) | 3 ( 3.2%) | 6 ( 3.4%) |
| >0% increase from baseline at Week 20 | 17 ( 20.0%) | 16 ( 17.2%) | 33 ( 18.5%) |
| <50% reduction from baseline at Week 20 | 46 ( 54.1%) | 59 ( 63.4%) | 105 ( 59.0%) |
| Return Rate (%) | 84% | 93% | 88% |
| Number of Subjects in Risk | 75 | 90 | 165 |
| Return Rate in Risk (%) | 96% | 97% | 96% |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
Data Extracted: CRF data: 01JUL2019
Source: t-tss24-overall.sas V.03.05 Output file: t-tss24-gba-db.pdf 29AUG2023: 9:54

Table 2.0701: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 84 | 93 | 177 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 1 ( 1.2%) | 0 | 1 ( 0.6%) |
| Responder, n(%) | 21 ( 25.0%) | 33 ( 35.5%) | 54 ( 30.5%) |
| 95% Exact CI | 0.1619, 0.3564 | 0.2583, 0.4609 | 0.2382, 0.3786 |
| Proportion Difference - Stratified CMH Method (95% CI) | -0.12 (-0.26, 0.02) | | |
| p-value | 0.11 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | -0.10 (-0.24, 0.03) | | |
| p-value | 0.13 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | -0.10 (-0.25, 0.04) | | |
| p-value | 0.14 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 1.42 (0.90, 2.25) | | |
| p-value [1] | 0.14 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 1.65 (0.86, 3.17) | | |
| p-value [1] | 0.13 | | |
| Unadjusted Absolute Risk Difference (95% CI) | -0.10 (-0.24, 0.03) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | 1.47 (0.93, 2.32) | | |
| p-value [2] | 0.098 | | |

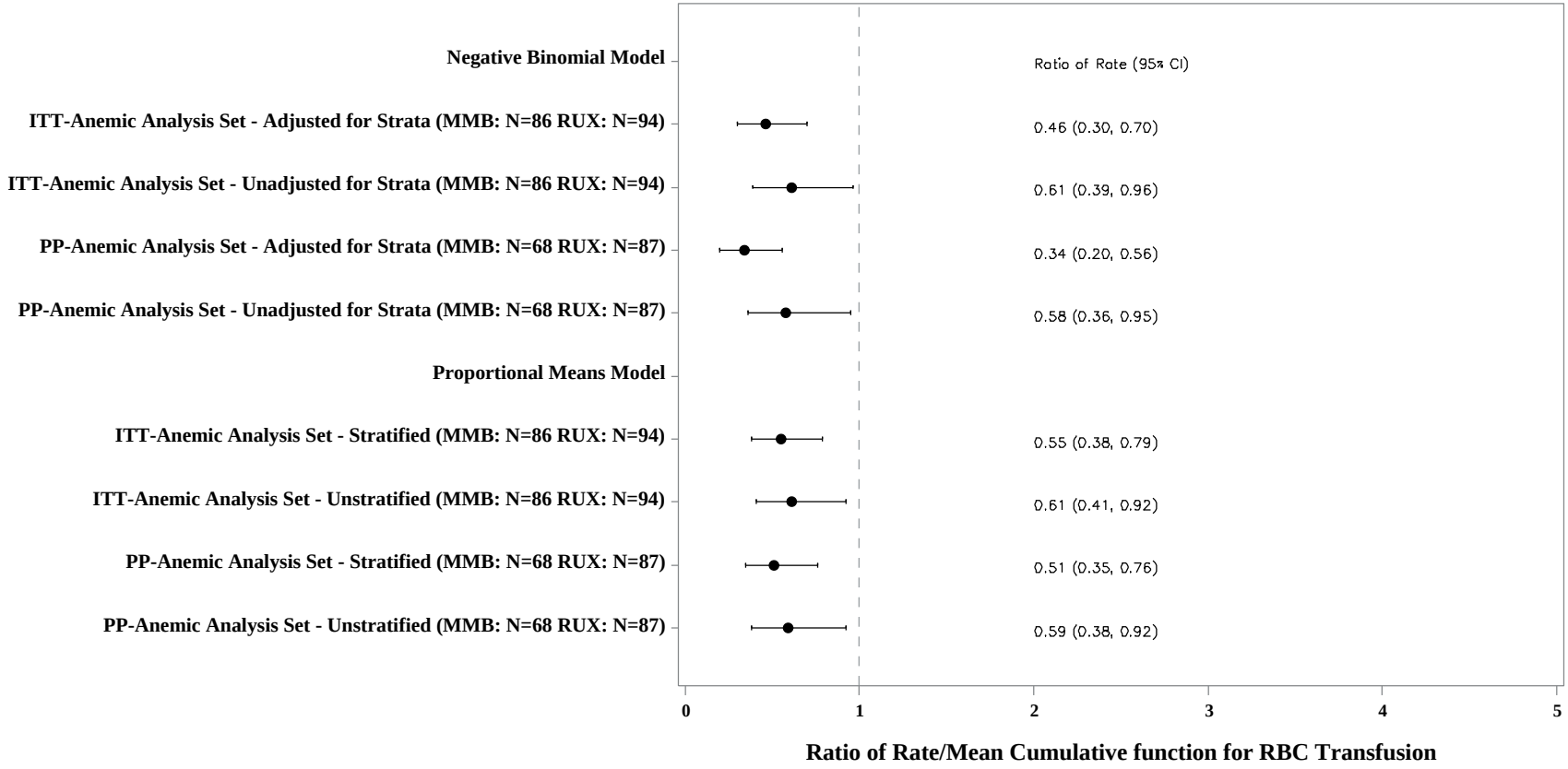
ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
Data Extracted: CRF data: 01JUL2019
Source: t-tss24-overall.sas V.03.05 Output file: t-tss24-gba-db.pdf 29AUG2023: 9:54

Table 2.0701: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Non-Responder, n(%) | 63 ( 75.0%) | 60 ( 64.5%) | 123 ( 69.5%) |
| Last participation date < Day 162 in DB Phase | 16 ( 19.0%) | 8 ( 8.6%) | 24 ( 13.6%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 3 ( 3.6%) | 6 ( 6.5%) | 9 ( 5.1%) |
| >0% increase from baseline at Week 24 | 17 ( 20.2%) | 13 ( 14.0%) | 30 ( 16.9%) |
| <50% reduction from baseline at Week 24 | 43 ( 51.2%) | 46 ( 49.5%) | 89 ( 50.3%) |
| Return Rate (%) | 78% | 85% | 82% |
| Number of Subjects in Risk | 70 | 86 | 156 |
| Return Rate in Risk (%) | 96% | 93% | 94% |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
Data Extracted: CRF data: 01JUL2019
Source: t-tss24-overall.sas V.03.05 Output file: t-tss24-gba-db.pdf 29AUG2023: 9:54

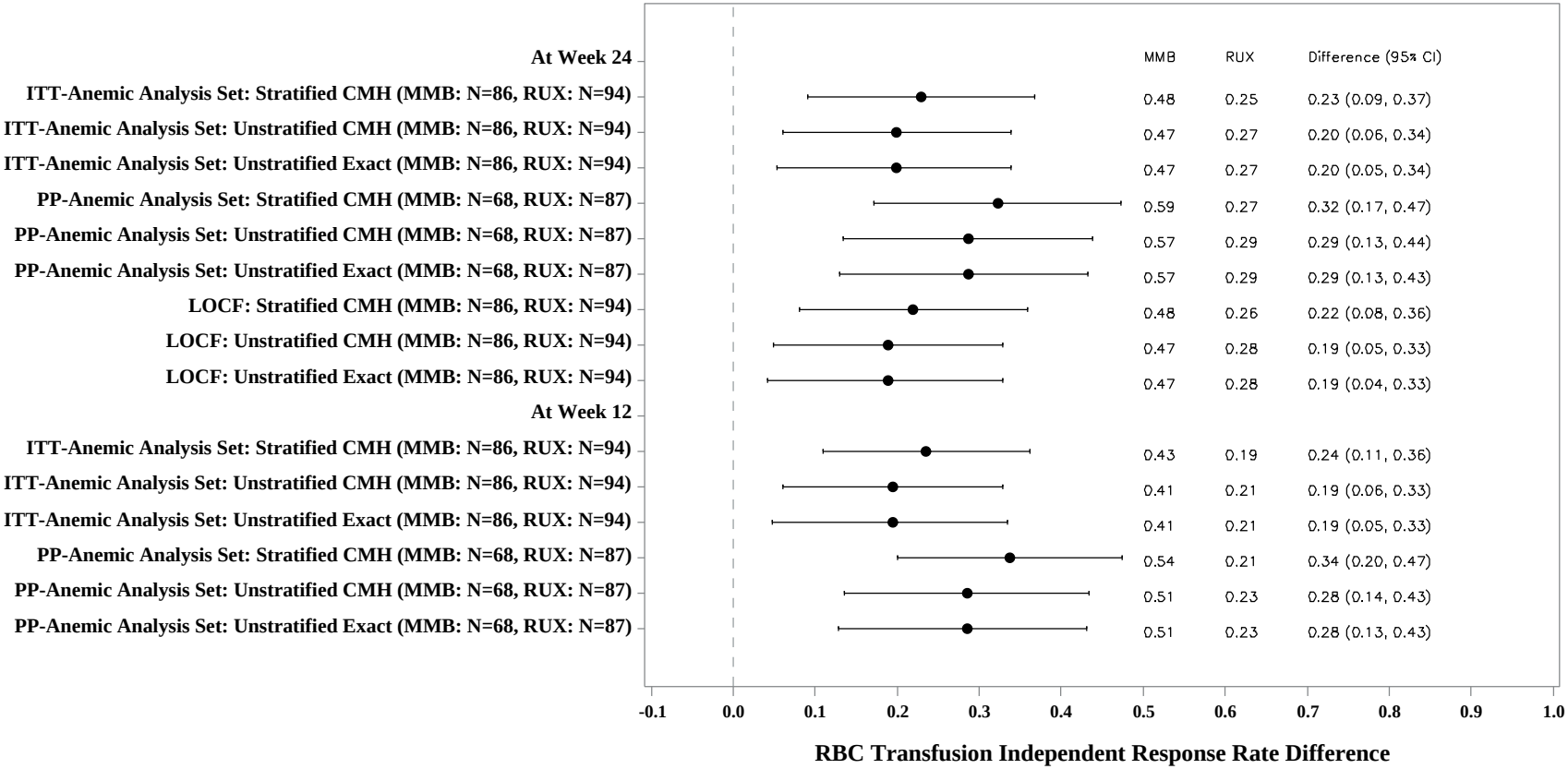
Figure 2.1301: Forest plot of Primary and Sensitivity Analysis of Rate of RBC Transfusion
Double-Blind Phase
ITT-Anemic and PP-Anemic Analysis Sets
All strata combined




ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Adjusted analysis for strata is used for negative binomial method. Stratified analysis is used for Proportional Means method.
To the left of the reference line favors MMB, to the right favors RUX.
RBC = Red Blood Cell; PP = Per-Protocol; CI = Confidence Interval

Data Extracted: CRF data: 01JUL2019
Source: g-rbc-forest.sas V.03.05 Output file: g-rbc-forest.pdf 30AUG2023:12:26

Figure 2.2001: Forest plot of Primary and Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic and PP-Anemic Analysis Sets
All strata combined




ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
To the right of the reference line favors MMB, to the left favors RUX.
CMH = Cochran-Mantel-Haenszel; LOCF = Last Observation Carried Forward; PP=Per-Protocol; CI= Confidence Interval
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
Source: g-rbc-ti-forest.sas V.03.05 Output file: g-rbc-ti-forest.pdf 30AUG2023:12:25

Table 2.2701: Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| **All Strata Combined** | | | |
| **RBC Transfusion Independent Rate at Week 24** | | | |
| Responder, n(%) | 40 ( 46.5%) | 25 ( 26.6%) | 65 ( 36.1%) |
| 95% Exact CI | 0.3568, 0.5759 | 0.1801, 0.3671 | 0.2910, 0.4359 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.23 (0.09, 0.37) | | |
| p-value | 0.001 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.20 (0.06, 0.34) | | |
| p-value | 0.005 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.20 (0.05, 0.34) | | |
| p-value | 0.008 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 0.57 (0.38, 0.86) | | |
| p-value [1] | 0.007 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 0.42 (0.22, 0.78) | | |
| p-value [1] | 0.006 | | |
| Unadjusted Absolute Risk Difference (95% CI) | 0.20 (0.06, 0.34) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | 0.53 (0.35, 0.78) | | |
| p-value [2] | 0.001 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell
[1] p-values were from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019
Source: t-tf-ti-resp.sas V.03.05 Output file: t-rbcti24-resp.pdf 29AUG2023: 9:35

Table 2.2701: Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Non-Responder, n(%) | 46 ( 53.5%) | 69 ( 73.4%) | 115 ( 63.9%) |
| Transfusion (except bleeding) in the last 12 weeks | 26 ( 30.2%) | 55 ( 58.5%) | 81 ( 45.0%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 23 ( 26.7%) | 45 ( 47.9%) | 68 ( 37.8%) |
| Last Participation date < Day 162 in DB phase | 16 ( 18.6%) | 8 ( 8.5%) | 24 ( 13.3%) |
| Other | 12 ( 14.0%) | 25 ( 26.6%) | 37 ( 20.6%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell
[1] p-values were from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019

Table 2.2701: Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| **RBC Transfusion Independent Rate at Week 12** | | | |
| Responder, n(%) | 35 ( 40.7%) | 20 ( 21.3%) | 55 ( 30.6%) |
| 95% Exact CI | 0.3022, 0.5183 | 0.1351, 0.3093 | 0.2392, 0.3784 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.24 (0.11, 0.36) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.19 (0.06, 0.33) | | |
| p-value | 0.004 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.19 (0.05, 0.33) | | |
| p-value | 0.006 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 0.52 (0.33, 0.83) | | |
| p-value [1] | 0.006 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 0.39 (0.20, 0.76) | | |
| p-value [1] | 0.005 | | |
| Unadjusted Absolute Risk Difference (95% CI) | 0.19 (0.06, 0.33) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | 0.46 (0.30, 0.70) | | |
| p-value [2] | <0.001 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell
[1] p-values were from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019
Source: t-tf-ti-resp.sas V.03.05 Output file: t-rbcti24-resp.pdf 29AUG2023: 9:35

Table 2.2701: Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Non-Responder, n(%) | 51 ( 59.3%) | 74 ( 78.7%) | 125 ( 69.4%) |
| Transfusion (except bleeding) in the last 12 weeks | 38 ( 44.2%) | 67 ( 71.3%) | 105 ( 58.3%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 23 ( 26.7%) | 64 ( 68.1%) | 87 ( 48.3%) |
| Last Participation date < Day 78 in DB phase | 10 ( 11.6%) | 0 | 10 ( 5.6%) |
| Other | 13 ( 15.1%) | 22 ( 23.4%) | 35 ( 19.4%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell
[1] p-values were from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019
Source: t-tf-ti-resp.sas V.03.05 Output file: t-rbcti24-resp.pdf 29AUG2023: 9:35

Table 2.2701: Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Platelet Count < 100X10E9/L | 10 | 9 | 19 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 5 ( 50.0%) | 2 ( 22.2%) | 7 ( 36.8%) |
| 95% Exact CI | 0.1871, 0.8129 | 0.0281, 0.6001 | 0.1629, 0.6164 |
| Proportion Difference (95% CI) | 0.28 (-0.17, 0.72) | | |
| Proportion Difference using Exact Method (95% CI) | 0.28 (-0.18, 0.67) | | |
| Non-Responder, n(%) | 5 ( 50.0%) | 7 ( 77.8%) | 12 ( 63.2%) |
| Transfusion (except bleeding) in the last 12 weeks | 4 ( 40.0%) | 4 ( 44.4%) | 8 ( 42.1%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 3 ( 30.0%) | 4 ( 44.4%) | 7 ( 36.8%) |
| Last Participation date < Day 162 in DB phase | 1 ( 10.0%) | 3 ( 33.3%) | 4 ( 21.1%) |
| Other | 1 ( 10.0%) | 3 ( 33.3%) | 4 ( 21.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell
[1] p-values were from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019
Source: t-tf-ti-resp.sas V.03.05 Output file: t-rbcti24-resp.pdf 29AUG2023: 9:35

Table 2.2701: Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| **RBC Transfusion Independent Rate at Week 12** | | | |
| Responder, n(%) | 2 ( 20.0%) | 1 ( 11.1%) | 3 ( 15.8%) |
| 95% Exact CI | 0.0252, 0.5561 | 0.0028, 0.4825 | 0.0338, 0.3958 |
| Proportion Difference (95% CI) | 0.09 (-0.28, 0.46) | | |
| Proportion Difference using Exact Method (95% CI) | 0.09 (-0.33, 0.51) | | |
| Non-Responder, n(%) | 8 ( 80.0%) | 8 ( 88.9%) | 16 ( 84.2%) |
| Transfusion (except bleeding) in the last 12 weeks | 7 ( 70.0%) | 7 ( 77.8%) | 14 ( 73.7%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 4 ( 40.0%) | 6 ( 66.7%) | 10 ( 52.6%) |
| Last Participation date < Day 78 in DB phase | 1 ( 10.0%) | 0 | 1 ( 5.3%) |
| Other | 1 ( 10.0%) | 4 ( 44.4%) | 5 ( 26.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell
[1] p-values were from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019
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Table 2.2701: Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 20 | 18 | 38 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 7 ( 35.0%) | 4 ( 22.2%) | 11 ( 28.9%) |
| 95% Exact CI | 0.1539, 0.5922 | 0.0641, 0.4764 | 0.1542, 0.4590 |
| Proportion Difference (95% CI) | 0.13 (-0.17, 0.42) | | |
| Proportion Difference using Exact Method (95% CI) | 0.13 (-0.19, 0.43) | | |
| Non-Responder, n(%) | 13 ( 65.0%) | 14 ( 77.8%) | 27 ( 71.1%) |
| Transfusion (except bleeding) in the last 12 weeks | 7 ( 35.0%) | 7 ( 38.9%) | 14 ( 36.8%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 6 ( 30.0%) | 7 ( 38.9%) | 13 ( 34.2%) |
| Last Participation date < Day 162 in DB phase | 5 ( 25.0%) | 4 ( 22.2%) | 9 ( 23.7%) |
| Other | 4 ( 20.0%) | 4 ( 22.2%) | 8 ( 21.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell
[1] p-values were from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019
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Table 2.2701: Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| **RBC Transfusion Independent Rate at Week 12** | | | |
| Responder, n(%) | 4 ( 20.0%) | 2 ( 11.1%) | 6 ( 15.8%) |
| 95% Exact CI | 0.0573, 0.4366 | 0.0138, 0.3471 | 0.0602, 0.3125 |
| Proportion Difference (95% CI) | 0.09 (-0.16, 0.33) | | |
| Proportion Difference using Exact Method (95% CI) | 0.09 (-0.24, 0.39) | | |
| Non-Responder, n(%) | 16 ( 80.0%) | 16 ( 88.9%) | 32 ( 84.2%) |
| Transfusion (except bleeding) in the last 12 weeks | 12 ( 60.0%) | 14 ( 77.8%) | 26 ( 68.4%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 9 ( 45.0%) | 14 ( 77.8%) | 23 ( 60.5%) |
| Last Participation date < Day 78 in DB phase | 3 ( 15.0%) | 0 | 3 ( 7.9%) |
| Other | 6 ( 30.0%) | 9 ( 50.0%) | 15 ( 39.5%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell
[1] p-values were from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

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Table 2.2701: Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence Yes and Platelet Count > 200X10E9/L | 19 | 16 | 35 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 4 ( 21.1%) | 2 ( 12.5%) | 6 ( 17.1%) |
| 95% Exact CI | 0.0605, 0.4557 | 0.0155, 0.3835 | 0.0656, 0.3365 |
| Proportion Difference (95% CI) | 0.09 (-0.18, 0.35) | | |
| Proportion Difference using Exact Method (95% CI) | 0.09 (-0.25, 0.40) | | |
| Non-Responder, n(%) | 15 ( 78.9%) | 14 ( 87.5%) | 29 ( 82.9%) |
| Transfusion (except bleeding) in the last 12 weeks | 10 ( 52.6%) | 14 ( 87.5%) | 24 ( 68.6%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 10 ( 52.6%) | 12 ( 75.0%) | 22 ( 62.9%) |
| Last Participation date < Day 162 in DB phase | 3 ( 15.8%) | 0 | 3 ( 8.6%) |
| Other | 6 ( 31.6%) | 7 ( 43.8%) | 13 ( 37.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell
[1] p-values were from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

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Table 2.2701: Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| **RBC Transfusion Independent Rate at Week 12** | | | |
| Responder, n(%) | 3 ( 15.8%) | 1 ( 6.3%) | 4 ( 11.4%) |
| 95% Exact CI | 0.0338, 0.3958 | 0.0016, 0.3023 | 0.0320, 0.2674 |
| Proportion Difference (95% CI) | 0.10 (-0.13, 0.32) | | |
| Proportion Difference using Exact Method (95% CI) | 0.10 (-0.24, 0.41) | | |
| Non-Responder, n(%) | 16 ( 84.2%) | 15 ( 93.8%) | 31 ( 88.6%) |
| Transfusion (except bleeding) in the last 12 weeks | 12 ( 63.2%) | 14 ( 87.5%) | 26 ( 74.3%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 8 ( 42.1%) | 14 ( 87.5%) | 22 ( 62.9%) |
| Last Participation date < Day 78 in DB phase | 3 ( 15.8%) | 0 | 3 ( 8.6%) |
| Other | 5 ( 26.3%) | 7 ( 43.8%) | 12 ( 34.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell
[1] p-values were from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019
Source: t-tf-ti-resp.sas V.03.05 Output file: t-rbcti24-resp.pdf 29AUG2023: 9:35

Table 2.2701: Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Platelet Count < 100X10E9/L | 3 | 4 | 7 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 2 ( 66.7%) | 0 | 2 ( 28.6%) |
| 95% Exact CI | 0.0943, 0.9916 | 0.0000, 0.6024 | 0.0367, 0.7096 |
| Proportion Difference (95% CI) | 0.67 (-0.09, 1.42) | | |
| Proportion Difference using Exact Method (95% CI) | 0.67 (-0.17, 0.99) | | |
| Non-Responder, n(%) | 1 ( 33.3%) | 4 (100.0%) | 5 ( 71.4%) |
| Transfusion (except bleeding) in the last 12 weeks | 1 ( 33.3%) | 2 ( 50.0%) | 3 ( 42.9%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 0 | 2 ( 50.0%) | 2 ( 28.6%) |
| Last Participation date < Day 162 in DB phase | 0 | 1 ( 25.0%) | 1 ( 14.3%) |
| Other | 0 | 0 | 0 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell
[1] p-values were from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019
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Table 2.2701: Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| **RBC Transfusion Independent Rate at Week 12** | | | |
| Responder, n(%) | 2 ( 66.7%) | 1 ( 25.0%) | 3 ( 42.9%) |
| 95% Exact CI | 0.0943, 0.9916 | 0.0063, 0.8059 | 0.0990, 0.8159 |
| Proportion Difference (95% CI) | 0.42 (-0.43, 1.26) | | |
| Proportion Difference using Exact Method (95% CI) | 0.42 (-0.40, 0.92) | | |
| Non-Responder, n(%) | 1 ( 33.3%) | 3 ( 75.0%) | 4 ( 57.1%) |
| Transfusion (except bleeding) in the last 12 weeks | 1 ( 33.3%) | 3 ( 75.0%) | 4 ( 57.1%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 1 ( 33.3%) | 2 ( 50.0%) | 3 ( 42.9%) |
| Last Participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Other | 0 | 0 | 0 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell
[1] p-values were from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

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Table 2.2701: Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 5 | | | |
| Transfusion Dependence No and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 16 | 16 | 32 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 11 ( 68.8%) | 4 ( 25.0%) | 15 ( 46.9%) |
| 95% Exact CI | 0.4134, 0.8898 | 0.0727, 0.5238 | 0.2909, 0.6526 |
| Proportion Difference (95% CI) | 0.44 (0.11, 0.76) | | |
| Proportion Difference using Exact Method (95% CI) | 0.44 (0.07, 0.73) | | |
| Non-Responder, n(%) | 5 ( 31.3%) | 12 ( 75.0%) | 17 ( 53.1%) |
| Transfusion (except bleeding) in the last 12 weeks | 2 ( 12.5%) | 11 ( 68.8%) | 13 ( 40.6%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 3 ( 18.8%) | 7 ( 43.8%) | 10 ( 31.3%) |
| Last Participation date < Day 162 in DB phase | 2 ( 12.5%) | 0 | 2 ( 6.3%) |
| Other | 1 ( 6.3%) | 4 ( 25.0%) | 5 ( 15.6%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell
[1] p-values were from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019

Table 2.2701: Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| **RBC Transfusion Independent Rate at Week 12** | | | |
| Responder, n(%) | 13 ( 81.3%) | 3 ( 18.8%) | 16 ( 50.0%) |
| 95% Exact CI | 0.5435, 0.9595 | 0.0405, 0.4565 | 0.3189, 0.6811 |
| Proportion Difference (95% CI) | 0.63 (0.34, 0.91) | | |
| Proportion Difference using Exact Method (95% CI) | 0.63 (0.27, 0.86) | | |
| Non-Responder, n(%) | 3 ( 18.8%) | 13 ( 81.3%) | 16 ( 50.0%) |
| Transfusion (except bleeding) in the last 12 weeks | 1 ( 6.3%) | 11 ( 68.8%) | 12 ( 37.5%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 0 | 11 ( 68.8%) | 11 ( 34.4%) |
| Last Participation date < Day 78 in DB phase | 1 ( 6.3%) | 0 | 1 ( 3.1%) |
| Other | 1 ( 6.3%) | 1 ( 6.3%) | 2 ( 6.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell
[1] p-values were from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

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Table 2.2701: Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 6 | | | |
| Transfusion Dependence No and Platelet Count > 200X10E9/L | 18 | 31 | 49 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 11 ( 61.1%) | 13 ( 41.9%) | 24 ( 49.0%) |
| 95% Exact CI | 0.3575, 0.8270 | 0.2455, 0.6092 | 0.3442, 0.6366 |
| Proportion Difference (95% CI) | 0.19 (-0.10, 0.48) | | |
| Proportion Difference using Exact Method (95% CI) | 0.19 (-0.10, 0.46) | | |
| Non-Responder, n(%) | 7 ( 38.9%) | 18 ( 58.1%) | 25 ( 51.0%) |
| Transfusion (except bleeding) in the last 12 weeks | 2 ( 11.1%) | 17 ( 54.8%) | 19 ( 38.8%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 1 ( 5.6%) | 13 ( 41.9%) | 14 ( 28.6%) |
| Last Participation date < Day 162 in DB phase | 5 ( 27.8%) | 0 | 5 ( 10.2%) |
| Other | 0 | 7 ( 22.6%) | 7 ( 14.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell
[1] p-values were from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019
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Table 2.2701: Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| **RBC Transfusion Independent Rate at Week 12** | | | |
| Responder, n(%) | 11 ( 61.1%) | 12 ( 38.7%) | 23 ( 46.9%) |
| 95% Exact CI | 0.3575, 0.8270 | 0.2185, 0.5781 | 0.3253, 0.6173 |
| Proportion Difference (95% CI) | 0.22 (-0.07, 0.51) | | |
| Proportion Difference using Exact Method (95% CI) | 0.22 (-0.07, 0.49) | | |
| Non-Responder, n(%) | 7 ( 38.9%) | 19 ( 61.3%) | 26 ( 53.1%) |
| Transfusion (except bleeding) in the last 12 weeks | 5 ( 27.8%) | 18 ( 58.1%) | 23 ( 46.9%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 1 ( 5.6%) | 17 ( 54.8%) | 18 ( 36.7%) |
| Last Participation date < Day 78 in DB phase | 2 ( 11.1%) | 0 | 2 ( 4.1%) |
| Other | 0 | 1 ( 3.2%) | 1 ( 2.0%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell
[1] p-values were from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.

Data Extracted: CRF data: 01JUL2019
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Table 2.3201: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| All Strata Combined | | | |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 40 ( 46.5%) | 26 ( 27.7%) | 66 ( 36.7%) |
| 95% Exact CI | 0.3568, 0.5759 | 0.1893, 0.3785 | 0.2962, 0.4416 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.22 (0.08, 0.36) | | |
| p-value | 0.002 | | |
| Proportion Difference - Unstratified Method (95% CI) | 0.19 (0.05, 0.33) | | |
| p-value | 0.008 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.19 (0.04, 0.33) | | |
| p-value | 0.013 | | |
| Non-Responder, n(%) | 46 ( 53.5%) | 68 ( 72.3%) | 114 ( 63.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3201: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Platelet Count < 100X10E9/L | 10 | 9 | 19 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 5 ( 50.0%) | 2 ( 22.2%) | 7 ( 36.8%) |
| 95% Exact CI | 0.1871, 0.8129 | 0.0281, 0.6001 | 0.1629, 0.6164 |
| Proportion Difference (95% CI) | 0.28 (-0.17, 0.72) | | |
| Proportion Difference using Exact Method (95% CI) | 0.28 (-0.18, 0.67) | | |
| Non-Responder, n(%) | 5 ( 50.0%) | 7 ( 77.8%) | 12 ( 63.2%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3201: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 20 | 18 | 38 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 7 ( 35.0%) | 4 ( 22.2%) | 11 ( 28.9%) |
| 95% Exact CI | 0.1539, 0.5922 | 0.0641, 0.4764 | 0.1542, 0.4590 |
| Proportion Difference (95% CI) | 0.13 (-0.17, 0.42) | | |
| Proportion Difference using Exact Method (95% CI) | 0.13 (-0.19, 0.43) | | |
| Non-Responder, n(%) | 13 ( 65.0%) | 14 ( 77.8%) | 27 ( 71.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3201: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence Yes and Platelet Count > 200X10E9/L | 19 | 16 | 35 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 4 ( 21.1%) | 2 ( 12.5%) | 6 ( 17.1%) |
| 95% Exact CI | 0.0605, 0.4557 | 0.0155, 0.3835 | 0.0656, 0.3365 |
| Proportion Difference (95% CI) | 0.09 (-0.18, 0.35) | | |
| Proportion Difference using Exact Method (95% CI) | 0.09 (-0.25, 0.40) | | |
| Non-Responder, n(%) | 15 ( 78.9%) | 14 ( 87.5%) | 29 ( 82.9%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3201: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Platelet Count < 100X10E9/L | 3 | 4 | 7 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 2 ( 66.7%) | 1 ( 25.0%) | 3 ( 42.9%) |
| 95% Exact CI | 0.0943, 0.9916 | 0.0063, 0.8059 | 0.0990, 0.8159 |
| Proportion Difference (95% CI) | 0.42 (-0.43, 1.26) | | |
| Proportion Difference using Exact Method (95% CI) | 0.42 (-0.40, 0.92) | | |
| Non-Responder, n(%) | 1 ( 33.3%) | 3 ( 75.0%) | 4 ( 57.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3201: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 5 | | | |
| Transfusion Dependence No and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 16 | 16 | 32 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 11 ( 68.8%) | 4 ( 25.0%) | 15 ( 46.9%) |
| 95% Exact CI | 0.4134, 0.8898 | 0.0727, 0.5238 | 0.2909, 0.6526 |
| Proportion Difference (95% CI) | 0.44 (0.11, 0.76) | | |
| Proportion Difference using Exact Method (95% CI) | 0.44 (0.07, 0.73) | | |
| Non-Responder, n(%) | 5 ( 31.3%) | 12 ( 75.0%) | 17 ( 53.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3201: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 6 | | | |
| Transfusion Dependence No and Platelet Count > 200X10E9/L | 18 | 31 | 49 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 11 ( 61.1%) | 13 ( 41.9%) | 24 ( 49.0%) |
| 95% Exact CI | 0.3575, 0.8270 | 0.2455, 0.6092 | 0.3442, 0.6366 |
| Proportion Difference (95% CI) | 0.19 (-0.10, 0.48) | | |
| Proportion Difference using Exact Method (95% CI) | 0.19 (-0.10, 0.46) | | |
| Non-Responder, n(%) | 7 ( 38.9%) | 18 ( 58.1%) | 25 ( 51.0%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3202: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| All Strata Combined | | | |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 39 ( 57.4%) | 25 ( 28.7%) | 64 ( 41.3%) |
| 95% Exact CI | 0.4477, 0.6928 | 0.1954, 0.3943 | 0.3345, 0.4947 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.32 (0.17, 0.47) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified Method (95% CI) | 0.29 (0.13, 0.44) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.29 (0.13, 0.43) | | |
| p-value | <0.001 | | |
| Non-Responder, n(%) | 29 ( 42.6%) | 62 ( 71.3%) | 91 ( 58.7%) |
| Transfusion(except bleeding) in the last 12 weeks | 23 ( 33.8%) | 52 ( 59.8%) | 75 ( 48.4%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 21 ( 30.9%) | 42 ( 48.3%) | 63 ( 40.6%) |
| Last Participation date < Day 162 in DB phase | 2 ( 2.9%) | 4 ( 4.6%) | 6 ( 3.9%) |
| Other | 11 ( 16.2%) | 23 ( 26.4%) | 34 ( 21.9%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3202: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| RBC Transfusion Independent Rate at Week 12 | | | |
| Responder, n(%) | 35 ( 51.5%) | 20 ( 23.0%) | 55 ( 35.5%) |
| 95% Exact CI | 0.3903, 0.6378 | 0.1464, 0.3325 | 0.2797, 0.4356 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.34 (0.20, 0.47) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified Method (95% CI) | 0.28 (0.14, 0.43) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.28 (0.13, 0.43) | | |
| p-value | <0.001 | | |
| Non-Responder, n(%) | 33 ( 48.5%) | 67 ( 77.0%) | 100 ( 64.5%) |
| Transfusion(except bleeding) in the last 12 weeks | 32 ( 47.1%) | 62 ( 71.3%) | 94 ( 60.6%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 19 ( 27.9%) | 59 ( 67.8%) | 78 ( 50.3%) |
| Last Participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Other | 9 ( 13.2%) | 17 ( 19.5%) | 26 ( 16.8%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3202: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Platelet Count < 100X10E9/L | 8 | 6 | 14 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 4 ( 50.0%) | 2 ( 33.3%) | 6 ( 42.9%) |
| 95% Exact CI | 0.1570, 0.8430 | 0.0433, 0.7772 | 0.1766, 0.7114 |
| Proportion Difference (95% CI) | 0.17 (-0.39, 0.73) | | |
| Proportion Difference using Exact Method (95% CI) | 0.17 (-0.38, 0.64) | | |
| Non-Responder, n(%) | 4 ( 50.0%) | 4 ( 66.7%) | 8 ( 57.1%) |
| Transfusion(except bleeding) in the last 12 weeks | 4 ( 50.0%) | 3 ( 50.0%) | 7 ( 50.0%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 3 ( 37.5%) | 3 ( 50.0%) | 6 ( 42.9%) |
| Last Participation date < Day 162 in DB phase | 0 | 1 ( 16.7%) | 1 ( 7.1%) |
| Other | 1 ( 12.5%) | 2 ( 33.3%) | 3 ( 21.4%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3202: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| RBC Transfusion Independent Rate at Week 12 | | | |
| Responder, n(%) | 2 ( 25.0%) | 1 ( 16.7%) | 3 ( 21.4%) |
| 95% Exact CI | 0.0319, 0.6509 | 0.0042, 0.6412 | 0.0466, 0.5080 |
| Proportion Difference (95% CI) | 0.08 (-0.41, 0.57) | | |
| Proportion Difference using Exact Method (95% CI) | 0.08 (-0.43, 0.58) | | |
| Non-Responder, n(%) | 6 ( 75.0%) | 5 ( 83.3%) | 11 ( 78.6%) |
| Transfusion(except bleeding) in the last 12 weeks | 6 ( 75.0%) | 5 ( 83.3%) | 11 ( 78.6%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 4 ( 50.0%) | 4 ( 66.7%) | 8 ( 57.1%) |
| Last Participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Other | 1 ( 12.5%) | 2 ( 33.3%) | 3 ( 21.4%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3202: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 15 | 16 | 31 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 7 ( 46.7%) | 4 ( 25.0%) | 11 ( 35.5%) |
| 95% Exact CI | 0.2127, 0.7341 | 0.0727, 0.5238 | 0.1923, 0.5463 |
| Proportion Difference (95% CI) | 0.22 (-0.13, 0.56) | | |
| Proportion Difference using Exact Method (95% CI) | 0.22 (-0.16, 0.53) | | |
| Non-Responder, n(%) | 8 ( 53.3%) | 12 ( 75.0%) | 20 ( 64.5%) |
| Transfusion(except bleeding) in the last 12 weeks | 7 ( 46.7%) | 7 ( 43.8%) | 14 ( 45.2%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 6 ( 40.0%) | 7 ( 43.8%) | 13 ( 41.9%) |
| Last Participation date < Day 162 in DB phase | 0 | 2 ( 12.5%) | 2 ( 6.5%) |
| Other | 4 ( 26.7%) | 4 ( 25.0%) | 8 ( 25.8%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3202: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| RBC Transfusion Independent Rate at Week 12 | | | |
| Responder, n(%) | 4 ( 26.7%) | 2 ( 12.5%) | 6 ( 19.4%) |
| 95% Exact CI | 0.0779, 0.5510 | 0.0155, 0.3835 | 0.0745, 0.3747 |
| Proportion Difference (95% CI) | 0.14 (-0.15, 0.44) | | |
| Proportion Difference using Exact Method (95% CI) | 0.14 (-0.22, 0.45) | | |
| Non-Responder, n(%) | 11 ( 73.3%) | 14 ( 87.5%) | 25 ( 80.6%) |
| Transfusion(except bleeding) in the last 12 weeks | 10 ( 66.7%) | 13 ( 81.3%) | 23 ( 74.2%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 7 ( 46.7%) | 13 ( 81.3%) | 20 ( 64.5%) |
| Last Participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Other | 5 ( 33.3%) | 7 ( 43.8%) | 12 ( 38.7%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3202: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence Yes and Platelet Count > 200X10E9/L | 14 | 14 | 28 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 4 ( 28.6%) | 2 ( 14.3%) | 6 ( 21.4%) |
| 95% Exact CI | 0.0839, 0.5810 | 0.0178, 0.4281 | 0.0830, 0.4095 |
| Proportion Difference (95% CI) | 0.14 (-0.18, 0.46) | | |
| Proportion Difference using Exact Method (95% CI) | 0.14 (-0.26, 0.51) | | |
| Non-Responder, n(%) | 10 ( 71.4%) | 12 ( 85.7%) | 22 ( 78.6%) |
| Transfusion(except bleeding) in the last 12 weeks | 8 ( 57.1%) | 12 ( 85.7%) | 20 ( 71.4%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 8 ( 57.1%) | 10 ( 71.4%) | 18 ( 64.3%) |
| Last Participation date < Day 162 in DB phase | 0 | 0 | 0 |
| Other | 5 ( 35.7%) | 6 ( 42.9%) | 11 ( 39.3%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3202: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| RBC Transfusion Independent Rate at Week 12 | | | |
| Responder, n(%) | 3 ( 21.4%) | 1 ( 7.1%) | 4 ( 14.3%) |
| 95% Exact CI | 0.0466, 0.5080 | 0.0018, 0.3387 | 0.0403, 0.3267 |
| Proportion Difference (95% CI) | 0.14 (-0.14, 0.42) | | |
| Proportion Difference using Exact Method (95% CI) | 0.14 (-0.26, 0.51) | | |
| Non-Responder, n(%) | 11 ( 78.6%) | 13 ( 92.9%) | 24 ( 85.7%) |
| Transfusion(except bleeding) in the last 12 weeks | 11 ( 78.6%) | 12 ( 85.7%) | 23 ( 82.1%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 7 ( 50.0%) | 12 ( 85.7%) | 19 ( 67.9%) |
| Last Participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Other | 3 ( 21.4%) | 6 ( 42.9%) | 9 ( 32.1%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3202: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Platelet Count < 100X10E9/L | 3 | 4 | 7 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 2 ( 66.7%) | 0 | 2 ( 28.6%) |
| 95% Exact CI | 0.0943, 0.9916 | 0.0000, 0.6024 | 0.0367, 0.7096 |
| Proportion Difference (95% CI) | 0.67 (-0.09, 1.42) | | |
| Proportion Difference using Exact Method (95% CI) | 0.67 (-0.17, 0.99) | | |
| Non-Responder, n(%) | 1 ( 33.3%) | 4 (100.0%) | 5 ( 71.4%) |
| Transfusion(except bleeding) in the last 12 weeks | 1 ( 33.3%) | 2 ( 50.0%) | 3 ( 42.9%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 0 | 2 ( 50.0%) | 2 ( 28.6%) |
| Last Participation date < Day 162 in DB phase | 0 | 1 ( 25.0%) | 1 ( 14.3%) |
| Other | 0 | 0 | 0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019
Source: t-sen-ppt-rbcti24-gba.sas V.03.05 Output file: t-sen-ppt-rbcti24-gba.pdf 29AUG2023: 9:46

Table 2.3202: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| RBC Transfusion Independent Rate at Week 12 | | | |
| Responder, n(%) | 2 ( 66.7%) | 1 ( 25.0%) | 3 ( 42.9%) |
| 95% Exact CI | 0.0943, 0.9916 | 0.0063, 0.8059 | 0.0990, 0.8159 |
| Proportion Difference (95% CI) | 0.42 (-0.43, 1.26) | | |
| Proportion Difference using Exact Method (95% CI) | 0.42 (-0.40, 0.92) | | |
| Non-Responder, n(%) | 1 ( 33.3%) | 3 ( 75.0%) | 4 ( 57.1%) |
| Transfusion(except bleeding) in the last 12 weeks | 1 ( 33.3%) | 3 ( 75.0%) | 4 ( 57.1%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 1 ( 33.3%) | 2 ( 50.0%) | 3 ( 42.9%) |
| Last Participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Other | 0 | 0 | 0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3202: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 5 | | | |
| Transfusion Dependence No and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 14 | 16 | 30 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 11 ( 78.6%) | 4 ( 25.0%) | 15 ( 50.0%) |
| 95% Exact CI | 0.4920, 0.9534 | 0.0727, 0.5238 | 0.3130, 0.6870 |
| Proportion Difference (95% CI) | 0.54 (0.22, 0.86) | | |
| Proportion Difference using Exact Method (95% CI) | 0.54 (0.18, 0.79) | | |
| Non-Responder, n(%) | 3 ( 21.4%) | 12 ( 75.0%) | 15 ( 50.0%) |
| Transfusion(except bleeding) in the last 12 weeks | 2 ( 14.3%) | 11 ( 68.8%) | 13 ( 43.3%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 3 ( 21.4%) | 7 ( 43.8%) | 10 ( 33.3%) |
| Last Participation date < Day 162 in DB phase | 0 | 0 | 0 |
| Other | 1 ( 7.1%) | 4 ( 25.0%) | 5 ( 16.7%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3202: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| RBC Transfusion Independent Rate at Week 12 | | | |
| Responder, n(%) | 13 ( 92.9%) | 3 ( 18.8%) | 16 ( 53.3%) |
| 95% Exact CI | 0.6613, 0.9982 | 0.0405, 0.4565 | 0.3433, 0.7166 |
| Proportion Difference (95% CI) | 0.74 (0.48, 1.00) | | |
| Proportion Difference using Exact Method (95% CI) | 0.74 (0.42, 0.92) | | |
| Non-Responder, n(%) | 1 ( 7.1%) | 13 ( 81.3%) | 14 ( 46.7%) |
| Transfusion(except bleeding) in the last 12 weeks | 1 ( 7.1%) | 11 ( 68.8%) | 12 ( 40.0%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 0 | 11 ( 68.8%) | 11 ( 36.7%) |
| Last Participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Other | 0 | 1 ( 6.3%) | 1 ( 3.3%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3202: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 6 | | | |
| Transfusion Dependence No and Platelet Count > 200X10E9/L | 14 | 31 | 45 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 11 ( 78.6%) | 13 ( 41.9%) | 24 ( 53.3%) |
| 95% Exact CI | 0.4920, 0.9534 | 0.2455, 0.6092 | 0.3787, 0.6834 |
| Proportion Difference (95% CI) | 0.37 (0.08, 0.66) | | |
| Proportion Difference using Exact Method (95% CI) | 0.37 (0.05, 0.65) | | |
| Non-Responder, n(%) | 3 ( 21.4%) | 18 ( 58.1%) | 21 ( 46.7%) |
| Transfusion(except bleeding) in the last 12 weeks | 1 ( 7.1%) | 17 ( 54.8%) | 18 ( 40.0%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 1 ( 7.1%) | 13 ( 41.9%) | 14 ( 31.1%) |
| Last Participation date < Day 162 in DB phase | 2 ( 14.3%) | 0 | 2 ( 4.4%) |
| Other | 0 | 7 ( 22.6%) | 7 ( 15.6%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.3202: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| RBC Transfusion Independent Rate at Week 12 | | | |
| Responder, n(%) | 11 ( 78.6%) | 12 ( 38.7%) | 23 ( 51.1%) |
| 95% Exact CI | 0.4920, 0.9534 | 0.2185, 0.5781 | 0.3577, 0.6630 |
| Proportion Difference (95% CI) | 0.40 (0.11, 0.69) | | |
| Proportion Difference using Exact Method (95% CI) | 0.40 (0.08, 0.67) | | |
| Non-Responder, n(%) | 3 ( 21.4%) | 19 ( 61.3%) | 22 ( 48.9%) |
| Transfusion(except bleeding) in the last 12 weeks | 3 ( 21.4%) | 18 ( 58.1%) | 21 ( 46.7%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 0 | 17 ( 54.8%) | 17 ( 37.8%) |
| Last Participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Other | 0 | 1 ( 3.2%) | 1 ( 2.2%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 01JUL2019

Table 2.1701: Sensitivity Analysis of Rate of RBC Transfusion
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| All Strata Combined | | | |
| RBC transfusion Rate in DB phase (units/month) | | | |
| N | 68 | 87 | 155 |
| Mean (SD) | 0.9 (1.41) | 1.6 (1.71) | 1.3 (1.61) |
| Median | 0.2 | 1.1 | 0.7 |
| Q1, Q3 | 0.0, 1.3 | 0.2, 2.2 | 0.0, 2.1 |
| Min, Max | 0.0, 6.4 | 0.0, 8.2 | 0.0, 8.2 |
| Negative Binomial Model | | | |
| Adjusted for Strata | | | |
| Rate of RBC transfusion with 95% CI (units/month) | 0.64 (0.44, 0.92) | 1.89 (1.31, 2.72) | |
| Ratio of Rate for RBC Transfusion with 95% CI | 0.34 (0.20, 0.56) | - | - |
| p-value | < 0.001 | - | - |
| Unadjusted for Strata | | | |
| Rate of RBC transfusion with 95% CI (units/month) | 0.90 (0.62, 1.31) | 1.55 (1.13, 2.14) | |
| Ratio of Rate for RBC Transfusion with 95% CI | 0.58 (0.36, 0.95) | - | - |
| p-value | 0.030 | - | - |
| Proportional Means Model - Supportive Analysis | | | |
| Stratified | | | |
| Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI | 0.51 (0.35, 0.76) | - | - |
| p-value | < 0.001 | - | - |
| Unstratified | | | |
| Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI | 0.59 (0.38, 0.92) | - | - |
| p-value | 0.018 | - | - |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
Source: t-sen-rbc24.sas V.03.05 Output file: t-sen-rbc24.pdf 29AUG2023: 9:43

Table 2.1701: Sensitivity Analysis of Rate of RBC Transfusion
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Total number of RBC transfusion unit in DB phase | | | |
| N | 68 | 87 | 155 |
| Mean (SD) | 5.0 (7.91) | 8.5 (9.13) | 7.0 (8.76) |
| Median | 1.0 | 6.0 | 4.0 |
| Q1, Q3 | 0.0, 7.0 | 1.0, 12.0 | 0.0, 12.0 |
| Min, Max | 0.0, 36.0 | 0.0, 38.0 | 0.0, 38.0 |
| Duration of DB phase (months) | | | |
| N | 68 | 87 | 155 |
| Mean (SD) | 5.55 (0.116) | 5.53 (0.187) | 5.54 (0.159) |
| Median | 5.55 | 5.55 | 5.55 |
| Q1, Q3 | 5.52, 5.59 | 5.49, 5.59 | 5.52, 5.59 |
| Min, Max | 4.80, 5.78 | 4.63, 5.78 | 4.63, 5.78 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
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Table 2.1701: Sensitivity Analysis of Rate of RBC Transfusion
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence at Baseline = 'Yes' and Platelet Count < 100*10E9/L | | | |
| RBC transfusion Rate in DB phase (units/month) | | | |
| N | 8 | 6 | 14 |
| Mean (SD) | 1.8 (1.63) | 2.8 (3.27) | 2.2 (2.40) |
| Median | 1.5 | 1.8 | 1.5 |
| Q1, Q3 | 0.4, 3.4 | 0.2, 4.6 | 0.4, 3.4 |
| Min, Max | 0.0, 4.3 | 0.0, 8.2 | 0.0, 8.2 |
| Negative Binomial Model | | | |
| Rate of RBC transfusion with 95% CI (units/month) | 1.85 (0.78, 4.39) | 2.76 (1.03, 7.44) | |
| Ratio of Rate for RBC Transfusion with 95% CI | 0.67 (0.18, 2.49) | - | - |
| Proportional Means Model - Supportive Analysis | | | |
| Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI | 0.72 (0.26, 1.95) | - | - |
| Total number of RBC transfusion in stratum 1 in DB phase | | | |
| N | 8 | 6 | 14 |
| Mean (SD) | 10.3 (9.08) | 14.2 (15.78) | 11.9 (12.01) |
| Median | 8.5 | 10.0 | 8.5 |
| Q1, Q3 | 2.0, 18.5 | 1.0, 26.0 | 2.0, 19.0 |
| Min, Max | 0.0, 24.0 | 0.0, 38.0 | 0.0, 38.0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
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Table 2.1701: Sensitivity Analysis of Rate of RBC Transfusion
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Duration of the DB phase (months) in stratum 1 | | | |
| N | 8 | 6 | 14 |
| Mean (SD) | 5.55 (0.048) | 5.40 (0.378) | 5.48 (0.249) |
| Median | 5.55 | 5.54 | 5.55 |
| Q1, Q3 | 5.50, 5.59 | 5.49, 5.59 | 5.49, 5.59 |
| Min, Max | 5.49, 5.62 | 4.63, 5.62 | 4.63, 5.62 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
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Table 2.1701: Sensitivity Analysis of Rate of RBC Transfusion
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence at Baseline = 'Yes' and Platelet Count >= 100*10E9/L | | | |
| RBC transfusion Rate in DB phase (units/month) | | | |
| N | 15 | 16 | 31 |
| Mean (SD) | 1.4 (1.94) | 1.9 (2.04) | 1.7 (1.97) |
| Median | 0.7 | 0.7 | 0.7 |
| Q1, Q3 | 0.0, 1.4 | 0.6, 2.8 | 0.0, 2.3 |
| Min, Max | 0.0, 6.4 | 0.0, 6.9 | 0.0, 6.9 |
| Negative Binomial Model | | | |
| Rate of RBC transfusion with 95% CI (units/month) | 1.42 (0.71, 2.85) | 1.87 (0.96, 3.66) | |
| Ratio of Rate for RBC Transfusion with 95% CI | 0.76 (0.29, 1.99) | - | - |
| Proportional Means Model - Supportive Analysis | | | |
| Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI | 0.76 (0.33, 1.78) | - | - |
| Total number of RBC transfusion in stratum 2 in DB phase | | | |
| N | 15 | 16 | 31 |
| Mean (SD) | 7.9 (10.87) | 10.1 (11.03) | 9.1 (10.83) |
| Median | 4.0 | 4.0 | 4.0 |
| Q1, Q3 | 0.0, 8.0 | 3.5, 15.5 | 0.0, 12.0 |
| Min, Max | 0.0, 36.0 | 0.0, 38.0 | 0.0, 38.0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
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Table 2.1701: Sensitivity Analysis of Rate of RBC Transfusion
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Duration of the DB phase (months) in stratum 2 | | | |
| N | 15 | 16 | 31 |
| Mean (SD) | 5.57 (0.032) | 5.45 (0.231) | 5.51 (0.175) |
| Median | 5.55 | 5.52 | 5.55 |
| Q1, Q3 | 5.55, 5.59 | 5.45, 5.55 | 5.52, 5.59 |
| Min, Max | 5.52, 5.65 | 4.67, 5.68 | 4.67, 5.68 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
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Table 2.1701: Sensitivity Analysis of Rate of RBC Transfusion
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence at Baseline = 'Yes' and Platelet Count > 200*10E9/L | | | |
| RBC transfusion Rate in DB phase (units/month) | | | |
| N | 14 | 14 | 28 |
| Mean (SD) | 1.4 (1.34) | 2.5 (1.75) | 2.0 (1.62) |
| Median | 0.8 | 2.2 | 1.6 |
| Q1, Q3 | 0.4, 2.5 | 1.1, 4.2 | 0.4, 3.1 |
| Min, Max | 0.0, 3.6 | 0.0, 5.2 | 0.0, 5.2 |
| Negative Binomial Model | | | |
| Rate of RBC transfusion with 95% CI (units/month) | 1.42 (0.85, 2.37) | 2.48 (1.51, 4.09) | |
| Ratio of Rate for RBC Transfusion with 95% CI | 0.57 (0.28, 1.17) | - | - |
| Proportional Means Model - Supportive Analysis | | | |
| Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI | 0.58 (0.32, 1.04) | - | - |
| Total number of RBC transfusion in stratum 3 in DB phase | | | |
| N | 14 | 14 | 28 |
| Mean (SD) | 7.9 (7.48) | 13.8 (9.79) | 10.9 (9.05) |
| Median | 4.5 | 12.0 | 9.0 |
| Q1, Q3 | 2.0, 14.0 | 6.0, 24.0 | 2.0, 17.5 |
| Min, Max | 0.0, 20.0 | 0.0, 29.0 | 0.0, 29.0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
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Table 2.1701: Sensitivity Analysis of Rate of RBC Transfusion
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Duration of the DB phase (months) in stratum 3 | | | |
| N | 14 | 14 | 28 |
| Mean (SD) | 5.57 (0.049) | 5.55 (0.104) | 5.56 (0.081) |
| Median | 5.59 | 5.54 | 5.55 |
| Q1, Q3 | 5.55, 5.62 | 5.49, 5.59 | 5.52, 5.59 |
| Min, Max | 5.45, 5.65 | 5.32, 5.78 | 5.32, 5.78 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
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Table 2.1701: Sensitivity Analysis of Rate of RBC Transfusion
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence at Baseline = 'No' and Platelet Count < 100*10E9/L | | | |
| RBC transfusion Rate in DB phase (units/month) | | | |
| N | 3 | 4 | 7 |
| Mean (SD) | 0.9 (1.53) | 1.1 (0.96) | 1.0 (1.12) |
| Median | 0.0 | 1.1 | 0.6 |
| Q1, Q3 | 0.0, 2.7 | 0.3, 1.9 | 0.0, 2.1 |
| Min, Max | 0.0, 2.7 | 0.0, 2.1 | 0.0, 2.7 |
| Negative Binomial Model | | | |
| Rate of RBC transfusion with 95% CI (units/month) | 0.89 (0.14, 5.72) | 1.07 (0.22, 5.33) | |
| Ratio of Rate for RBC Transfusion with 95% CI | 0.83 (0.07, 9.69) | - | - |
| Proportional Means Model - Supportive Analysis | | | |
| Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI | 0.77 (0.13, 4.56) | - | - |
| Total number of RBC transfusion in stratum 4 in DB phase | | | |
| N | 3 | 4 | 7 |
| Mean (SD) | 5.0 (8.66) | 6.0 (5.48) | 5.6 (6.35) |
| Median | 0.0 | 6.0 | 3.0 |
| Q1, Q3 | 0.0, 15.0 | 1.5, 10.5 | 0.0, 12.0 |
| Min, Max | 0.0, 15.0 | 0.0, 12.0 | 0.0, 15.0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
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Table 2.1701: Sensitivity Analysis of Rate of RBC Transfusion
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Duration of the DB phase (months) in stratum 4 | | | |
| N | 3 | 4 | 7 |
| Mean (SD) | 5.60 (0.050) | 5.38 (0.404) | 5.47 (0.309) |
| Median | 5.59 | 5.50 | 5.55 |
| Q1, Q3 | 5.55, 5.65 | 5.13, 5.63 | 5.45, 5.65 |
| Min, Max | 5.55, 5.65 | 4.80, 5.72 | 4.80, 5.72 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
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Table 2.1701: Sensitivity Analysis of Rate of RBC Transfusion
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 5 | | | |
| Transfusion Dependence at Baseline = 'No' and Platelet Count >= 100*10E9/L and <= 200*10E9/L | | | |
| RBC transfusion Rate in DB phase (units/month) | | | |
| N | 14 | 16 | 30 |
| Mean (SD) | 0.1 (0.30) | 1.1 (0.96) | 0.6 (0.87) |
| Median | 0.0 | 0.9 | 0.1 |
| Q1, Q3 | 0.0, 0.0 | 0.3, 1.8 | 0.0, 1.1 |
| Min, Max | 0.0, 1.1 | 0.0, 3.2 | 0.0, 3.2 |
| Negative Binomial Model | | | |
| Rate of RBC transfusion with 95% CI (units/month) | 0.09 (0.03, 0.24) | 1.08 (0.58, 2.02) | |
| Ratio of Rate for RBC Transfusion with 95% CI | 0.08 (0.03, 0.27) | - | - |
| Proportional Means Model - Supportive Analysis | | | |
| Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI | 0.08 (0.02, 0.45) | - | - |
| Total number of RBC transfusion in stratum 5 in DB phase | | | |
| N | 14 | 16 | 30 |
| Mean (SD) | 0.5 (1.61) | 6.0 (5.34) | 3.4 (4.87) |
| Median | 0.0 | 5.0 | 0.5 |
| Q1, Q3 | 0.0, 0.0 | 1.5, 10.0 | 0.0, 6.0 |
| Min, Max | 0.0, 6.0 | 0.0, 18.0 | 0.0, 18.0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
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Table 2.1701: Sensitivity Analysis of Rate of RBC Transfusion
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Duration of the DB phase (months) in stratum 5 | | | |
| N | 14 | 16 | 30 |
| Mean (SD) | 5.52 (0.060) | 5.57 (0.103) | 5.55 (0.088) |
| Median | 5.54 | 5.55 | 5.55 |
| Q1, Q3 | 5.45, 5.55 | 5.52, 5.60 | 5.49, 5.59 |
| Min, Max | 5.42, 5.62 | 5.39, 5.78 | 5.39, 5.78 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
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Table 2.1701: Sensitivity Analysis of Rate of RBC Transfusion
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 6 | | | |
| Transfusion Dependence at Baseline = 'No' and Platelet Count > 200*10E9/L | | | |
| RBC transfusion Rate in DB phase (units/month) | | | |
| N | 14 | 31 | 45 |
| Mean (SD) | 0.1 (0.23) | 1.0 (1.20) | 0.7 (1.09) |
| Median | 0.0 | 0.7 | 0.2 |
| Q1, Q3 | 0.0, 0.2 | 0.0, 1.5 | 0.0, 1.2 |
| Min, Max | 0.0, 0.8 | 0.0, 4.3 | 0.0, 4.3 |
| Negative Binomial Model | | | |
| Rate of RBC transfusion with 95% CI (units/month) | 0.11 (0.04, 0.30) | 1.04 (0.61, 1.77) | |
| Ratio of Rate for RBC Transfusion with 95% CI | 0.10 (0.03, 0.33) | - | - |
| Proportional Means Model - Supportive Analysis | | | |
| Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI | 0.10 (0.03, 0.31) | - | - |
| Total number of RBC transfusion in stratum 6 in DB phase | | | |
| N | 14 | 31 | 45 |
| Mean (SD) | 0.6 (1.16) | 5.8 (6.70) | 4.2 (6.08) |
| Median | 0.0 | 4.0 | 1.0 |
| Q1, Q3 | 0.0, 1.0 | 0.0, 8.0 | 0.0, 7.0 |
| Min, Max | 0.0, 4.0 | 0.0, 24.0 | 0.0, 24.0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
Source: t-sen-rbc24.sas V.03.05 Output file: t-sen-rbc24.pdf 29AUG2023: 9:43

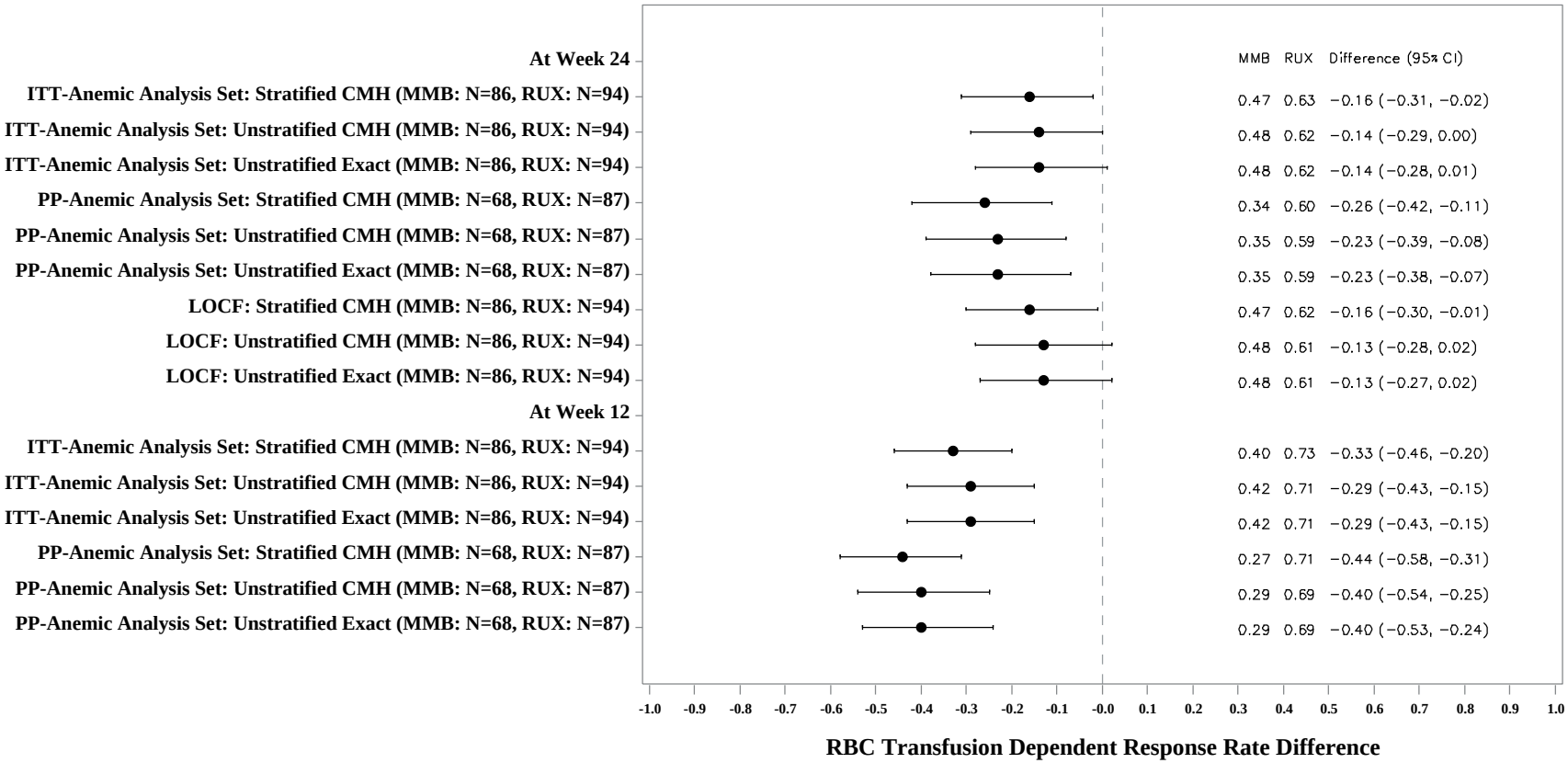
Table 2.1701: Sensitivity Analysis of Rate of RBC Transfusion
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Duration of the DB phase (months) in stratum 6 | | | |
| N | 14 | 31 | 45 |
| Mean (SD) | 5.50 (0.236) | 5.58 (0.101) | 5.56 (0.157) |
| Median | 5.55 | 5.55 | 5.55 |
| Q1, Q3 | 5.45, 5.59 | 5.52, 5.62 | 5.52, 5.62 |
| Min, Max | 4.80, 5.78 | 5.36, 5.78 | 4.80, 5.78 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
Source: t-sen-rbc24.sas V.03.05 Output file: t-sen-rbc24.pdf 29AUG2023: 9:43

Figure 2.2501: Forest plot of Primary and Sensitivity Analysis of RBC TD Response Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic and PP-Anemic Analysis Sets
All strata combined




ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
To the left of the reference line favors MMB, to the right favors RUX.
CMH = Cochran-Mantel-Haenszel; LOCF = Last Observation Carried Forward; PP=Per-Protocol; CI= Confidence Interval
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
Source: g-rbc-td-forest.sas V.03.05 Output file: g-rbc-td-forest.pdf 30AUG2023:12:25

Table 2.3801: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| **All Strata Combined** | | | |
| **RBC Transfusion Dependent Rate at Week 24** | | | |
| Not-Dependent, n(%) | 45 ( 52.3%) | 36 ( 38.3%) | 81 ( 45.0%) |
| Transfusion Requiring, n(%) | 5 ( 5.8%) | 11 ( 11.7%) | 16 ( 8.9%) |
| Transfusion Independent, n(%) | 40 ( 46.5%) | 25 ( 26.6%) | 65 ( 36.1%) |
| Dependent, n(%) | 41 ( 47.7%) | 58 ( 61.7%) | 99 ( 55.0%) |
| 95% Exact CI | 0.3679, 0.5873 | 0.5110, 0.7154 | 0.4742, 0.6241 |
| Proportion Difference - Stratified CMH Method (95% CI) | -0.16 (-0.31, -0.02) | | |
| p-value | 0.025 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | -0.14 (-0.29, 0.00) | | |
| p-value | 0.058 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | -0.14 (-0.28, 0.01) | | |
| p-value | 0.072 | | |
| Unadjusted Relative Risk (95% CI) | 0.77 (0.59, 1.01) | | |
| p-value [1] | 0.064 | | |
| Unadjusted Odds Ratio (95% CI) | 0.57 (0.31, 1.02) | | |
| p-value [1] | 0.060 | | |
| Unadjusted Absolute Risk Difference (95% CI) | -0.14 (-0.28, 0.00) | | |
| Adjusted Relative Risk (95% CI) [2] | 0.74 (0.57, 0.96) | | |
| p-value [2] | 0.024 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Data Extracted: CRF data: 01JUL2019

Table 2.3801: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| >=4 units transfused in the last 8 weeks | 14 ( 16.3%) | 29 ( 30.9%) | 43 ( 23.9%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 20 ( 23.3%) | 43 ( 45.7%) | 63 ( 35.0%) |
| Last Participation date < Day 162 in DB phase | 16 ( 18.6%) | 8 ( 8.5%) | 24 ( 13.3%) |
| Other | 11 ( 12.8%) | 26 ( 27.7%) | 37 ( 20.6%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Data Extracted: CRF data: 01JUL2019

Table 2.3801: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| **RBC Transfusion Dependent Rate at Week 12** | | | |
| Not-Dependent, n(%) | 50 ( 58.1%) | 27 ( 28.7%) | 77 ( 42.8%) |
| Transfusion Requiring, n(%) | 15 ( 17.4%) | 7 ( 7.4%) | 22 ( 12.2%) |
| Transfusion Independent, n(%) | 35 ( 40.7%) | 20 ( 21.3%) | 55 ( 30.6%) |
| Dependent, n(%) | 36 ( 41.9%) | 67 ( 71.3%) | 103 ( 57.2%) |
| 95% Exact CI | 0.3130, 0.5299 | 0.6102, 0.8014 | 0.4965, 0.6455 |
| Proportion Difference - Stratified CMH Method (95% CI) | -0.33 (-0.46, -0.20) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | -0.29 (-0.43, -0.15) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | -0.29 (-0.43, -0.15) | | |
| p-value | <0.001 | | |
| Unadjusted Relative Risk (95% CI) | 0.59 (0.44, 0.78) | | |
| p-value [1] | <0.001 | | |
| Unadjusted Odds Ratio (95% CI) | 0.29 (0.16, 0.54) | | |
| p-value [1] | <0.001 | | |
| Unadjusted Absolute Risk Difference (95% CI) | -0.29 (-0.43, -0.16) | | |
| Adjusted Relative Risk (95% CI) [2] | 0.55 (0.42, 0.72) | | |
| p-value [2] | <0.001 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Data Extracted: CRF data: 01JUL2019

Table 2.3801: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| >=4 units transfused in the last 8 weeks | 21 ( 24.4%) | 38 ( 40.4%) | 59 ( 32.8%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 21 ( 24.4%) | 63 ( 67.0%) | 84 ( 46.7%) |
| Last Participation date < Day 78 in DB phase | 10 ( 11.6%) | 0 | 10 ( 5.6%) |
| Other | 11 ( 12.8%) | 35 ( 37.2%) | 46 ( 25.6%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Data Extracted: CRF data: 01JUL2019

Table 2.3801: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Platelet Count < 100X10E9/L | 10 | 9 | 19 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Not-Dependent, n(%) | 5 ( 50.0%) | 2 ( 22.2%) | 7 ( 36.8%) |
| Transfusion Requiring, n(%) | 0 | 0 | 0 |
| Transfusion Independent, n(%) | 5 ( 50.0%) | 2 ( 22.2%) | 7 ( 36.8%) |
| Dependent, n(%) | 5 ( 50.0%) | 7 ( 77.8%) | 12 ( 63.2%) |
| 95% Exact CI | 0.1871, 0.8129 | 0.3999, 0.9719 | 0.3836, 0.8371 |
| Proportion Difference (95% CI) | -0.28 (-0.72, 0.17) | | |
| Proportion Difference using Exact Method (95% CI) | -0.28 (-0.67, 0.18) | | |
| >=4 units transfused in the last 8 weeks | 4 ( 40.0%) | 3 ( 33.3%) | 7 ( 36.8%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 3 ( 30.0%) | 4 ( 44.4%) | 7 ( 36.8%) |
| Last Participation date < Day 162 in DB phase | 1 ( 10.0%) | 3 ( 33.3%) | 4 ( 21.1%) |
| Other | 1 ( 10.0%) | 3 ( 33.3%) | 4 ( 21.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Data Extracted: CRF data: 01JUL2019

Table 2.3801: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| **RBC Transfusion Dependent Rate at Week 12** | | | |
| Not-Dependent, n(%) | 4 ( 40.0%) | 2 ( 22.2%) | 6 ( 31.6%) |
| Transfusion Requiring, n(%) | 2 ( 20.0%) | 1 ( 11.1%) | 3 ( 15.8%) |
| Transfusion Independent, n(%) | 2 ( 20.0%) | 1 ( 11.1%) | 3 ( 15.8%) |
| Dependent, n(%) | 6 ( 60.0%) | 7 ( 77.8%) | 13 ( 68.4%) |
| 95% Exact CI | 0.2624, 0.8784 | 0.3999, 0.9719 | 0.4345, 0.8742 |
| Proportion Difference (95% CI) | -0.18 (-0.62, 0.26) | | |
| Proportion Difference using Exact Method (95% CI) | -0.18 (-0.60, 0.27) | | |
| >=4 units transfused in the last 8 weeks | 5 ( 50.0%) | 6 ( 66.7%) | 11 ( 57.9%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 3 ( 30.0%) | 7 ( 77.8%) | 10 ( 52.6%) |
| Last Participation date < Day 78 in DB phase | 1 ( 10.0%) | 0 | 1 ( 5.3%) |
| Other | 1 ( 10.0%) | 4 ( 44.4%) | 5 ( 26.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Data Extracted: CRF data: 01JUL2019

Table 2.3801: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 20 | 18 | 38 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Not-Dependent, n(%) | 9 ( 45.0%) | 8 ( 44.4%) | 17 ( 44.7%) |
| Transfusion Requiring, n(%) | 2 ( 10.0%) | 4 ( 22.2%) | 6 ( 15.8%) |
| Transfusion Independent, n(%) | 7 ( 35.0%) | 4 ( 22.2%) | 11 ( 28.9%) |
| Dependent, n(%) | 11 ( 55.0%) | 10 ( 55.6%) | 21 ( 55.3%) |
| 95% Exact CI | 0.3153, 0.7694 | 0.3076, 0.7847 | 0.3830, 0.7138 |
| Proportion Difference (95% CI) | -0.01 (-0.33, 0.32) | | |
| Proportion Difference using Exact Method (95% CI) | -0.01 (-0.33, 0.31) | | |
| >=4 units transfused in the last 8 weeks | 3 ( 15.0%) | 3 ( 16.7%) | 6 ( 15.8%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 5 ( 25.0%) | 6 ( 33.3%) | 11 ( 28.9%) |
| Last Participation date < Day 162 in DB phase | 5 ( 25.0%) | 4 ( 22.2%) | 9 ( 23.7%) |
| Other | 3 ( 15.0%) | 5 ( 27.8%) | 8 ( 21.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Data Extracted: CRF data: 01JUL2019

Table 2.3801: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| **RBC Transfusion Dependent Rate at Week 12** | | | |
| Not-Dependent, n(%) | 7 ( 35.0%) | 3 ( 16.7%) | 10 ( 26.3%) |
| Transfusion Requiring, n(%) | 3 ( 15.0%) | 1 ( 5.6%) | 4 ( 10.5%) |
| Transfusion Independent, n(%) | 4 ( 20.0%) | 2 ( 11.1%) | 6 ( 15.8%) |
| Dependent, n(%) | 13 ( 65.0%) | 15 ( 83.3%) | 28 ( 73.7%) |
| 95% Exact CI | 0.4078, 0.8461 | 0.5858, 0.9642 | 0.5690, 0.8660 |
| Proportion Difference (95% CI) | -0.18 (-0.47, 0.10) | | |
| Proportion Difference using Exact Method (95% CI) | -0.18 (-0.48, 0.14) | | |
| >=4 units transfused in the last 8 weeks | 8 ( 40.0%) | 9 ( 50.0%) | 17 ( 44.7%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 9 ( 45.0%) | 15 ( 83.3%) | 24 ( 63.2%) |
| Last Participation date < Day 78 in DB phase | 3 ( 15.0%) | 0 | 3 ( 7.9%) |
| Other | 5 ( 25.0%) | 10 ( 55.6%) | 15 ( 39.5%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Data Extracted: CRF data: 01JUL2019

Table 2.3801: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence Yes and Platelet Count > 200X10E9/L | 19 | 16 | 35 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Not-Dependent, n(%) | 6 ( 31.6%) | 2 ( 12.5%) | 8 ( 22.9%) |
| Transfusion Requiring, n(%) | 2 ( 10.5%) | 0 | 2 ( 5.7%) |
| Transfusion Independent, n(%) | 4 ( 21.1%) | 2 ( 12.5%) | 6 ( 17.1%) |
| Dependent, n(%) | 13 ( 68.4%) | 14 ( 87.5%) | 27 ( 77.1%) |
| 95% Exact CI | 0.4345, 0.8742 | 0.6165, 0.9845 | 0.5986, 0.8958 |
| Proportion Difference (95% CI) | -0.19 (-0.47, 0.09) | | |
| Proportion Difference using Exact Method (95% CI) | -0.19 (-0.49, 0.15) | | |
| >=4 units transfused in the last 8 weeks | 5 ( 26.3%) | 10 ( 62.5%) | 15 ( 42.9%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 9 ( 47.4%) | 11 ( 68.8%) | 20 ( 57.1%) |
| Last Participation date < Day 162 in DB phase | 3 ( 15.8%) | 0 | 3 ( 8.6%) |
| Other | 7 ( 36.8%) | 7 ( 43.8%) | 14 ( 40.0%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Data Extracted: CRF data: 01JUL2019

Table 2.3801: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| **RBC Transfusion Dependent Rate at Week 12** | | | |
| Not-Dependent, n(%) | 9 ( 47.4%) | 2 ( 12.5%) | 11 ( 31.4%) |
| Transfusion Requiring, n(%) | 6 ( 31.6%) | 1 ( 6.3%) | 7 ( 20.0%) |
| Transfusion Independent, n(%) | 3 ( 15.8%) | 1 ( 6.3%) | 4 ( 11.4%) |
| Dependent, n(%) | 10 ( 52.6%) | 14 ( 87.5%) | 24 ( 68.6%) |
| 95% Exact CI | 0.2886, 0.7555 | 0.6165, 0.9845 | 0.5071, 0.8315 |
| Proportion Difference (95% CI) | -0.35 (-0.64, -0.06) | | |
| Proportion Difference using Exact Method (95% CI) | -0.35 (-0.62, -0.01) | | |
| >=4 units transfused in the last 8 weeks | 5 ( 26.3%) | 10 ( 62.5%) | 15 ( 42.9%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 7 ( 36.8%) | 13 ( 81.3%) | 20 ( 57.1%) |
| Last Participation date < Day 78 in DB phase | 3 ( 15.8%) | 0 | 3 ( 8.6%) |
| Other | 3 ( 15.8%) | 11 ( 68.8%) | 14 ( 40.0%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Data Extracted: CRF data: 01JUL2019

Table 2.3801: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Platelet Count < 100X10E9/L | 3 | 4 | 7 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Not-Dependent, n(%) | 2 ( 66.7%) | 0 | 2 ( 28.6%) |
| Transfusion Requiring, n(%) | 0 | 0 | 0 |
| Transfusion Independent, n(%) | 2 ( 66.7%) | 0 | 2 ( 28.6%) |
| Dependent, n(%) | 1 ( 33.3%) | 4 (100.0%) | 5 ( 71.4%) |
| 95% Exact CI | 0.0084, 0.9057 | 0.3976, 1.0000 | 0.2904, 0.9633 |
| Proportion Difference (95% CI) | -0.67 (-1.42, 0.09) | | |
| Proportion Difference using Exact Method (95% CI) | -0.67 (-0.99, 0.17) | | |
| >=4 units transfused in the last 8 weeks | 1 ( 33.3%) | 2 ( 50.0%) | 3 ( 42.9%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 0 | 2 ( 50.0%) | 2 ( 28.6%) |
| Last Participation date < Day 162 in DB phase | 0 | 1 ( 25.0%) | 1 ( 14.3%) |
| Other | 0 | 1 ( 25.0%) | 1 ( 14.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Data Extracted: CRF data: 01JUL2019
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Table 2.3801: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| **RBC Transfusion Dependent Rate at Week 12** | | | |
| Not-Dependent, n(%) | 2 ( 66.7%) | 3 ( 75.0%) | 5 ( 71.4%) |
| Transfusion Requiring, n(%) | 0 | 2 ( 50.0%) | 2 ( 28.6%) |
| Transfusion Independent, n(%) | 2 ( 66.7%) | 1 ( 25.0%) | 3 ( 42.9%) |
| Dependent, n(%) | 1 ( 33.3%) | 1 ( 25.0%) | 2 ( 28.6%) |
| 95% Exact CI | 0.0084, 0.9057 | 0.0063, 0.8059 | 0.0367, 0.7096 |
| Proportion Difference (95% CI) | 0.08 (-0.76, 0.93) | | |
| Proportion Difference using Exact Method (95% CI) | 0.08 (-0.63, 0.76) | | |
| >=4 units transfused in the last 8 weeks | 1 ( 33.3%) | 1 ( 25.0%) | 2 ( 28.6%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 1 ( 33.3%) | 1 ( 25.0%) | 2 ( 28.6%) |
| Last Participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Other | 0 | 0 | 0 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Data Extracted: CRF data: 01JUL2019

Table 2.3801: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 5 | | | |
| Transfusion Dependence No and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 16 | 16 | 32 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Not-Dependent, n(%) | 12 ( 75.0%) | 8 ( 50.0%) | 20 ( 62.5%) |
| Transfusion Requiring, n(%) | 1 ( 6.3%) | 4 ( 25.0%) | 5 ( 15.6%) |
| Transfusion Independent, n(%) | 11 ( 68.8%) | 4 ( 25.0%) | 15 ( 46.9%) |
| Dependent, n(%) | 4 ( 25.0%) | 8 ( 50.0%) | 12 ( 37.5%) |
| 95% Exact CI | 0.0727, 0.5238 | 0.2465, 0.7535 | 0.2110, 0.5631 |
| Proportion Difference (95% CI) | -0.25 (-0.59, 0.09) | | |
| Proportion Difference using Exact Method (95% CI) | -0.25 (-0.58, 0.13) | | |
| >=4 units transfused in the last 8 weeks | 0 | 3 ( 18.8%) | 3 ( 9.4%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 2 ( 12.5%) | 7 ( 43.8%) | 9 ( 28.1%) |
| Last Participation date < Day 162 in DB phase | 2 ( 12.5%) | 0 | 2 ( 6.3%) |
| Other | 0 | 1 ( 6.3%) | 1 ( 3.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Data Extracted: CRF data: 01JUL2019

Table 2.3801: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| **RBC Transfusion Dependent Rate at Week 12** | | | |
| Not-Dependent, n(%) | 14 ( 87.5%) | 4 ( 25.0%) | 18 ( 56.3%) |
| Transfusion Requiring, n(%) | 1 ( 6.3%) | 1 ( 6.3%) | 2 ( 6.3%) |
| Transfusion Independent, n(%) | 13 ( 81.3%) | 3 ( 18.8%) | 16 ( 50.0%) |
| Dependent, n(%) | 2 ( 12.5%) | 12 ( 75.0%) | 14 ( 43.8%) |
| 95% Exact CI | 0.0155, 0.3835 | 0.4762, 0.9273 | 0.2636, 0.6234 |
| Proportion Difference (95% CI) | -0.63 (-0.91, -0.34) | | |
| Proportion Difference using Exact Method (95% CI) | -0.63 (-0.86, -0.27) | | |
| >=4 units transfused in the last 8 weeks | 0 | 5 ( 31.3%) | 5 ( 15.6%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 0 | 10 ( 62.5%) | 10 ( 31.3%) |
| Last Participation date < Day 78 in DB phase | 1 ( 6.3%) | 0 | 1 ( 3.1%) |
| Other | 1 ( 6.3%) | 3 ( 18.8%) | 4 ( 12.5%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Data Extracted: CRF data: 01JUL2019
Source: t-rbctd24-gba.sas V.03.05 Output file: t-rbctd24-gba.pdf 29AUG2023: 9:36

Table 2.3801: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 6 | | | |
| Transfusion Dependence No and Platelet Count > 200X10E9/L | 18 | 31 | 49 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Not-Dependent, n(%) | 11 ( 61.1%) | 16 ( 51.6%) | 27 ( 55.1%) |
| Transfusion Requiring, n(%) | 0 | 3 ( 9.7%) | 3 ( 6.1%) |
| Transfusion Independent, n(%) | 11 ( 61.1%) | 13 ( 41.9%) | 24 ( 49.0%) |
| Dependent, n(%) | 7 ( 38.9%) | 15 ( 48.4%) | 22 ( 44.9%) |
| 95% Exact CI | 0.1730, 0.6425 | 0.3015, 0.6694 | 0.3067, 0.5977 |
| Proportion Difference (95% CI) | -0.09 (-0.39, 0.20) | | |
| Proportion Difference using Exact Method (95% CI) | -0.09 (-0.37, 0.20) | | |
| >=4 units transfused in the last 8 weeks | 1 ( 5.6%) | 8 ( 25.8%) | 9 ( 18.4%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 1 ( 5.6%) | 13 ( 41.9%) | 14 ( 28.6%) |
| Last Participation date < Day 162 in DB phase | 5 ( 27.8%) | 0 | 5 ( 10.2%) |
| Other | 0 | 9 ( 29.0%) | 9 ( 18.4%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Data Extracted: CRF data: 01JUL2019
Source: t-rbctd24-gba.sas V.03.05 Output file: t-rbctd24-gba.pdf 29AUG2023: 9:36

Table 2.3801: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| **RBC Transfusion Dependent Rate at Week 12** | | | |
| Not-Dependent, n(%) | 14 ( 77.8%) | 13 ( 41.9%) | 27 ( 55.1%) |
| Transfusion Requiring, n(%) | 3 ( 16.7%) | 1 ( 3.2%) | 4 ( 8.2%) |
| Transfusion Independent, n(%) | 11 ( 61.1%) | 12 ( 38.7%) | 23 ( 46.9%) |
| Dependent, n(%) | 4 ( 22.2%) | 18 ( 58.1%) | 22 ( 44.9%) |
| 95% Exact CI | 0.0641, 0.4764 | 0.3908, 0.7545 | 0.3067, 0.5977 |
| Proportion Difference (95% CI) | -0.36 (-0.63, -0.09) | | |
| Proportion Difference using Exact Method (95% CI) | -0.36 (-0.60, -0.07) | | |
| >=4 units transfused in the last 8 weeks | 2 ( 11.1%) | 7 ( 22.6%) | 9 ( 18.4%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 1 ( 5.6%) | 17 ( 54.8%) | 18 ( 36.7%) |
| Last Participation date < Day 78 in DB phase | 2 ( 11.1%) | 0 | 2 ( 4.1%) |
| Other | 1 ( 5.6%) | 7 ( 22.6%) | 8 ( 16.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline platelet count (<100, 100-200, >200 10^9/L) as covariates.
Data Extracted: CRF data: 01JUL2019

Table 2.4001: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| All Strata Combined | | | |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Non-Dependent, n(%) | 45 ( 52.3%) | 37 ( 39.4%) | 82 ( 45.6%) |
| Transfusion Requiring, n(%) | 5 ( 5.8%) | 11 ( 11.7%) | 16 ( 8.9%) |
| Transfusion Independent, n(%) | 40 ( 46.5%) | 26 ( 27.7%) | 66 ( 36.7%) |
| Dependent, n(%) | 41 ( 47.7%) | 57 ( 60.6%) | 98 ( 54.4%) |
| 95% Exact CI | 0.3679, 0.5873 | 0.5002, 0.7056 | 0.4687, 0.6187 |
| Proportion Difference - Stratified CMH Method (95% CI) | -0.16 (-0.30, -0.01) | | |
| p-value | 0.035 | | |
| Proportion Difference - Unstratified Method (95% CI) | -0.13 (-0.28, 0.02) | | |
| p-value | 0.081 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | -0.13 (-0.27, 0.02) | | |
| p-value | 0.100 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
LOCF = Last Observation Carried Forward
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
Source: t-tf-td-sen.sas V.03.05 Output file: t-sen-locf-rbctd24.pdf 29AUG2023: 9:35

Table 2.4001: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Platelet Count < 100X10E9/L | 10 | 9 | 19 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Non-Dependent, n(%) | 5 ( 50.0%) | 2 ( 22.2%) | 7 ( 36.8%) |
| Transfusion Requiring, n(%) | 0 | 0 | 0 |
| Transfusion Independent, n(%) | 5 ( 50.0%) | 2 ( 22.2%) | 7 ( 36.8%) |
| Dependent, n(%) | 5 ( 50.0%) | 7 ( 77.8%) | 12 ( 63.2%) |
| 95% Exact CI | 0.1871, 0.8129 | 0.3999, 0.9719 | 0.3836, 0.8371 |
| Proportion Difference (95% CI) | -0.28 (-0.72, 0.17) | | |
| Proportion Difference using Exact Method (95% CI) | -0.28 (-0.67, 0.18) | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
LOCF = Last Observation Carried Forward
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
Source: t-tf-td-sen.sas V.03.05 Output file: t-sen-locf-rbctd24.pdf 29AUG2023: 9:35

Table 2.4001: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 20 | 18 | 38 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Non-Dependent, n(%) | 9 ( 45.0%) | 8 ( 44.4%) | 17 ( 44.7%) |
| Transfusion Requiring, n(%) | 2 ( 10.0%) | 4 ( 22.2%) | 6 ( 15.8%) |
| Transfusion Independent, n(%) | 7 ( 35.0%) | 4 ( 22.2%) | 11 ( 28.9%) |
| Dependent, n(%) | 11 ( 55.0%) | 10 ( 55.6%) | 21 ( 55.3%) |
| 95% Exact CI | 0.3153, 0.7694 | 0.3076, 0.7847 | 0.3830, 0.7138 |
| Proportion Difference (95% CI) | -0.01 (-0.33, 0.32) | | |
| Proportion Difference using Exact Method (95% CI) | -0.01 (-0.33, 0.31) | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
LOCF = Last Observation Carried Forward
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019

Table 2.4001: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence Yes and Platelet Count > 200X10E9/L | 19 | 16 | 35 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Non-Dependent, n(%) | 6 ( 31.6%) | 2 ( 12.5%) | 8 ( 22.9%) |
| Transfusion Requiring, n(%) | 2 ( 10.5%) | 0 | 2 ( 5.7%) |
| Transfusion Independent, n(%) | 4 ( 21.1%) | 2 ( 12.5%) | 6 ( 17.1%) |
| Dependent, n(%) | 13 ( 68.4%) | 14 ( 87.5%) | 27 ( 77.1%) |
| 95% Exact CI | 0.4345, 0.8742 | 0.6165, 0.9845 | 0.5986, 0.8958 |
| Proportion Difference (95% CI) | -0.19 (-0.47, 0.09) | | |
| Proportion Difference using Exact Method (95% CI) | -0.19 (-0.49, 0.15) | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
LOCF = Last Observation Carried Forward
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
Source: t-tf-td-sen.sas V.03.05 Output file: t-sen-locf-rbctd24.pdf 29AUG2023: 9:35

Table 2.4001: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Platelet Count < 100X10E9/L | 3 | 4 | 7 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Non-Dependent, n(%) | 2 ( 66.7%) | 1 ( 25.0%) | 3 ( 42.9%) |
| Transfusion Requiring, n(%) | 0 | 0 | 0 |
| Transfusion Independent, n(%) | 2 ( 66.7%) | 1 ( 25.0%) | 3 ( 42.9%) |
| Dependent, n(%) | 1 ( 33.3%) | 3 ( 75.0%) | 4 ( 57.1%) |
| 95% Exact CI | 0.0084, 0.9057 | 0.1941, 0.9937 | 0.1841, 0.9010 |
| Proportion Difference (95% CI) | -0.42 (-1.26, 0.43) | | |
| Proportion Difference using Exact Method (95% CI) | -0.42 (-0.92, 0.40) | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
LOCF = Last Observation Carried Forward
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
Source: t-tf-td-sen.sas V.03.05 Output file: t-sen-locf-rbctd24.pdf 29AUG2023: 9:35

Table 2.4001: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 5 | | | |
| Transfusion Dependence No and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 16 | 16 | 32 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Non-Dependent, n(%) | 12 ( 75.0%) | 8 ( 50.0%) | 20 ( 62.5%) |
| Transfusion Requiring, n(%) | 1 ( 6.3%) | 4 ( 25.0%) | 5 ( 15.6%) |
| Transfusion Independent, n(%) | 11 ( 68.8%) | 4 ( 25.0%) | 15 ( 46.9%) |
| Dependent, n(%) | 4 ( 25.0%) | 8 ( 50.0%) | 12 ( 37.5%) |
| 95% Exact CI | 0.0727, 0.5238 | 0.2465, 0.7535 | 0.2110, 0.5631 |
| Proportion Difference (95% CI) | -0.25 (-0.59, 0.09) | | |
| Proportion Difference using Exact Method (95% CI) | -0.25 (-0.58, 0.13) | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
LOCF = Last Observation Carried Forward
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
Source: t-tf-td-sen.sas V.03.05 Output file: t-sen-locf-rbctd24.pdf 29AUG2023: 9:35

Table 2.4001: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 -- LOCF
Double-Blind Phase
ITT-Anemic Analysis Set

| | MMB (N=86) | RUX (N=94) | Total (N=180) |
|---|---|---|---|
| Stratum 6 | | | |
| Transfusion Dependence No and Platelet Count > 200X10E9/L | 18 | 31 | 49 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Non-Dependent, n(%) | 11 ( 61.1%) | 16 ( 51.6%) | 27 ( 55.1%) |
| Transfusion Requiring, n(%) | 0 | 3 ( 9.7%) | 3 ( 6.1%) |
| Transfusion Independent, n(%) | 11 ( 61.1%) | 13 ( 41.9%) | 24 ( 49.0%) |
| Dependent, n(%) | 7 ( 38.9%) | 15 ( 48.4%) | 22 ( 44.9%) |
| 95% Exact CI | 0.1730, 0.6425 | 0.3015, 0.6694 | 0.3067, 0.5977 |
| Proportion Difference (95% CI) | -0.09 (-0.39, 0.20) | | |
| Proportion Difference using Exact Method (95% CI) | -0.09 (-0.37, 0.20) | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel
LOCF = Last Observation Carried Forward
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
Source: t-tf-td-sen.sas V.03.05 Output file: t-sen-locf-rbctd24.pdf 29AUG2023: 9:35

Table 2.4002: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| All Strata Combined | | | |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Non-Dependent, n(%) | 44 ( 64.7%) | 36 ( 41.4%) | 80 ( 51.6%) |
| Transfusion Requiring, n(%) | 5 ( 7.4%) | 11 ( 12.6%) | 16 ( 10.3%) |
| Transfusion Independent, n(%) | 39 ( 57.4%) | 25 ( 28.7%) | 64 ( 41.3%) |
| Dependent, n(%) | 24 ( 35.3%) | 51 ( 58.6%) | 75 ( 48.4%) |
| 95% Exact CI | 0.2408, 0.4783 | 0.4755, 0.6908 | 0.4030, 0.5654 |
| Proportion Difference - Stratified CMH Method (95% CI) | -0.26(-0.42, -0.11) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified Method (95% CI) | -0.23 (-0.39, -0.08) | | |
| p-value | 0.003 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | -0.23 (-0.38, -0.07) | | |
| p-value | 0.006 | | |
| >=4 units transfused in the last 8 weeks | 12 ( 17.6%) | 26 ( 29.9%) | 38 ( 24.5%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 18 ( 26.5%) | 40 ( 46.0%) | 58 ( 37.4%) |
| Last Participation date < Day 162 in DB phase | 2 ( 2.9%) | 4 ( 4.6%) | 6 ( 3.9%) |
| Other | 9 ( 13.2%) | 26 ( 29.9%) | 35 ( 22.6%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
Source: t-tf-td-sen-ppt.sas V.03.05 Output file: t-sen-ppt-rbctd24.pdf 29AUG2023: 9:38

Table 2.4002: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| RBC Transfusion Dependent Rate at Week 12 | | | |
| Non-Dependent, n(%) | 48 ( 70.6%) | 27 ( 31.0%) | 75 ( 48.4%) |
| Transfusion Requiring, n(%) | 13 ( 19.1%) | 7 ( 8.0%) | 20 ( 12.9%) |
| Transfusion Independent, n(%) | 35 ( 51.5%) | 20 ( 23.0%) | 55 ( 35.5%) |
| Dependent, n(%) | 20 ( 29.4%) | 60 ( 69.0%) | 80 ( 51.6%) |
| 95% Exact CI | 0.1898, 0.4171 | 0.5814, 0.7845 | 0.4346, 0.5970 |
| Proportion Difference - Stratified CMH Method (95% CI) | -0.44 (-0.58, -0.31) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified Method (95% CI) | -0.40 (-0.54, -0.25) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | -0.40 (-0.53, -0.24) | | |
| p-value | <0.001 | | |
| >=4 units transfused in the last 8 weeks | 17 ( 25.0%) | 31 ( 35.6%) | 48 ( 31.0%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 17 ( 25.0%) | 56 ( 64.4%) | 73 ( 47.1%) |
| Last Participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Other | 7 ( 10.3%) | 30 ( 34.5%) | 37 ( 23.9%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
Source: t-tf-td-sen-ppt.sas V.03.05 Output file: t-sen-ppt-rbctd24.pdf 29AUG2023: 9:38

Table 2.4002: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Platelet Count < 100X10E9/L | 8 | 6 | 14 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Non-Dependent, n(%) | 4 ( 50.0%) | 2 ( 33.3%) | 6 ( 42.9%) |
| Transfusion Requiring, n(%) | 0 | 0 | 0 |
| Transfusion Independent, n(%) | 4 ( 50.0%) | 2 ( 33.3%) | 6 ( 42.9%) |
| Dependent, n(%) | 4 ( 50.0%) | 4 ( 66.7%) | 8 ( 57.1%) |
| 95% Exact CI | 0.1570, 0.8430 | 0.2228, 0.9567 | 0.2886, 0.8234 |
| Proportion Difference (95% CI) | -0.17 (-0.73, 0.39) | | |
| Proportion Difference using Exact Method (95% CI) | -0.17 (-0.64, 0.38) | | |
| >=4 units transfused in the last 8 weeks | 4 ( 50.0%) | 2 ( 33.3%) | 6 ( 42.9%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 3 ( 37.5%) | 3 ( 50.0%) | 6 ( 42.9%) |
| Last Participation date < Day 162 in DB phase | 0 | 1 ( 16.7%) | 1 ( 7.1%) |
| Other | 1 ( 12.5%) | 3 ( 50.0%) | 4 ( 28.6%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
Source: t-tf-td-sen-ppt.sas V.03.05 Output file: t-sen-ppt-rbctd24.pdf 29AUG2023: 9:38

Table 2.4002: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| RBC Transfusion Dependent Rate at Week 12 | | | |
| Non-Dependent, n(%) | 3 ( 37.5%) | 2 ( 33.3%) | 5 ( 35.7%) |
| Transfusion Requiring, n(%) | 1 ( 12.5%) | 1 ( 16.7%) | 2 ( 14.3%) |
| Transfusion Independent, n(%) | 2 ( 25.0%) | 1 ( 16.7%) | 3 ( 21.4%) |
| Dependent, n(%) | 5 ( 62.5%) | 4 ( 66.7%) | 9 ( 64.3%) |
| 95% Exact CI | 0.2449, 0.9148 | 0.2228, 0.9567 | 0.3514, 0.8724 |
| Proportion Difference (95% CI) | -0.04 (-0.60, 0.51) | | |
| Proportion Difference using Exact Method (95% CI) | -0.04 (-0.54, 0.48) | | |
| >=4 units transfused in the last 8 weeks | 5 ( 62.5%) | 3 ( 50.0%) | 8 ( 57.1%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 3 ( 37.5%) | 4 ( 66.7%) | 7 ( 50.0%) |
| Last Participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Other | 1 ( 12.5%) | 2 ( 33.3%) | 3 ( 21.4%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019

Table 2.4002: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 15 | 16 | 31 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Non-Dependent, n(%) | 9 ( 60.0%) | 8 ( 50.0%) | 17 ( 54.8%) |
| Transfusion Requiring, n(%) | 2 ( 13.3%) | 4 ( 25.0%) | 6 ( 19.4%) |
| Transfusion Independent, n(%) | 7 ( 46.7%) | 4 ( 25.0%) | 11 ( 35.5%) |
| Dependent, n(%) | 6 ( 40.0%) | 8 ( 50.0%) | 14 ( 45.2%) |
| 95% Exact CI | 0.1634, 0.6771 | 0.2465, 0.7535 | 0.2732, 0.6397 |
| Proportion Difference (95% CI) | -0.10 (-0.46, 0.26) | | |
| Proportion Difference using Exact Method (95% CI) | -0.10 (-0.45, 0.26) | | |
| >=4 units transfused in the last 8 weeks | 3 ( 20.0%) | 3 ( 18.8%) | 6 ( 19.4%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 5 ( 33.3%) | 6 ( 37.5%) | 11 ( 35.5%) |
| Last Participation date < Day 162 in DB phase | 0 | 2 ( 12.5%) | 2 ( 6.5%) |
| Other | 3 ( 20.0%) | 5 ( 31.3%) | 8 ( 25.8%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019

Table 2.4002: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| RBC Transfusion Dependent Rate at Week 12 | | | |
| Non-Dependent, n(%) | 7 ( 46.7%) | 3 ( 18.8%) | 10 ( 32.3%) |
| Transfusion Requiring, n(%) | 3 ( 20.0%) | 1 ( 6.3%) | 4 ( 12.9%) |
| Transfusion Independent, n(%) | 4 ( 26.7%) | 2 ( 12.5%) | 6 ( 19.4%) |
| Dependent, n(%) | 8 ( 53.3%) | 13 ( 81.3%) | 21 ( 67.7%) |
| 95% Exact CI | 0.2659, 0.7873 | 0.5435, 0.9595 | 0.4863, 0.8332 |
| Proportion Difference (95% CI) | -0.28 (-0.61, 0.05) | | |
| Proportion Difference using Exact Method (95% CI) | -0.28 (-0.58, 0.09) | | |
| >=4 units transfused in the last 8 weeks | 6 ( 40.0%) | 7 ( 43.8%) | 13 ( 41.9%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 7 ( 46.7%) | 13 ( 81.3%) | 20 ( 64.5%) |
| Last Participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Other | 3 ( 20.0%) | 9 ( 56.3%) | 12 ( 38.7%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019

Table 2.4002: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence Yes and Platelet Count > 200X10E9/L | 14 | 14 | 28 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Non-Dependent, n(%) | 6 ( 42.9%) | 2 ( 14.3%) | 8 ( 28.6%) |
| Transfusion Requiring, n(%) | 2 ( 14.3%) | 0 | 2 ( 7.1%) |
| Transfusion Independent, n(%) | 4 ( 28.6%) | 2 ( 14.3%) | 6 ( 21.4%) |
| Dependent, n(%) | 8 ( 57.1%) | 12 ( 85.7%) | 20 ( 71.4%) |
| 95% Exact CI | 0.2886, 0.8234 | 0.5719, 0.9822 | 0.5133, 0.8678 |
| Proportion Difference (95% CI) | -0.29 (-0.62, 0.05) | | |
| Proportion Difference using Exact Method (95% CI) | -0.29 (-0.63, 0.12) | | |
| >=4 units transfused in the last 8 weeks | 4 ( 28.6%) | 8 ( 57.1%) | 12 ( 42.9%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 7 ( 50.0%) | 9 ( 64.3%) | 16 ( 57.1%) |
| Last Participation date < Day 162 in DB phase | 0 | 0 | 0 |
| Other | 5 ( 35.7%) | 7 ( 50.0%) | 12 ( 42.9%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019

Table 2.4002: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| RBC Transfusion Dependent Rate at Week 12 | | | |
| Non-Dependent, n(%) | 8 ( 57.1%) | 2 ( 14.3%) | 10 ( 35.7%) |
| Transfusion Requiring, n(%) | 5 ( 35.7%) | 1 ( 7.1%) | 6 ( 21.4%) |
| Transfusion Independent, n(%) | 3 ( 21.4%) | 1 ( 7.1%) | 4 ( 14.3%) |
| Dependent, n(%) | 6 ( 42.9%) | 12 ( 85.7%) | 18 ( 64.3%) |
| 95% Exact CI | 0.1766, 0.7114 | 0.5719, 0.9822 | 0.4407, 0.8136 |
| Proportion Difference (95% CI) | -0.43 (-0.77, -0.09) | | |
| Proportion Difference using Exact Method (95% CI) | -0.43 (-0.74, -0.03) | | |
| >=4 units transfused in the last 8 weeks | 5 ( 35.7%) | 8 ( 57.1%) | 13 ( 46.4%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 6 ( 42.9%) | 11 ( 78.6%) | 17 ( 60.7%) |
| Last Participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Other | 3 ( 21.4%) | 9 ( 64.3%) | 12 ( 42.9%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019
Source: t-tf-td-sen-ppt.sas V.03.05 Output file: t-sen-ppt-rbctd24.pdf 29AUG2023: 9:38

Table 2.4002: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Platelet Count < 100X10E9/L | 3 | 4 | 7 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Non-Dependent, n(%) | 2 ( 66.7%) | 0 | 2 ( 28.6%) |
| Transfusion Requiring, n(%) | 0 | 0 | 0 |
| Transfusion Independent, n(%) | 2 ( 66.7%) | 0 | 2 ( 28.6%) |
| Dependent, n(%) | 1 ( 33.3%) | 4 (100.0%) | 5 ( 71.4%) |
| 95% Exact CI | 0.0084, 0.9057 | 0.3976, 1.0000 | 0.2904, 0.9633 |
| Proportion Difference (95% CI) | -0.67 (-1.42, 0.09) | | |
| Proportion Difference using Exact Method (95% CI) | -0.67 (-0.99, 0.17) | | |
| >=4 units transfused in the last 8 weeks | 1 ( 33.3%) | 2 ( 50.0%) | 3 ( 42.9%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 0 | 2 ( 50.0%) | 2 ( 28.6%) |
| Last Participation date < Day 162 in DB phase | 0 | 1 ( 25.0%) | 1 ( 14.3%) |
| Other | 0 | 1 ( 25.0%) | 1 ( 14.3%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019

Table 2.4002: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| RBC Transfusion Dependent Rate at Week 12 | | | |
| Non-Dependent, n(%) | 2 ( 66.7%) | 3 ( 75.0%) | 5 ( 71.4%) |
| Transfusion Requiring, n(%) | 0 | 2 ( 50.0%) | 2 ( 28.6%) |
| Transfusion Independent, n(%) | 2 ( 66.7%) | 1 ( 25.0%) | 3 ( 42.9%) |
| Dependent, n(%) | 1 ( 33.3%) | 1 ( 25.0%) | 2 ( 28.6%) |
| 95% Exact CI | 0.0084, 0.9057 | 0.0063, 0.8059 | 0.0367, 0.7096 |
| Proportion Difference (95% CI) | 0.08 (-0.76, 0.93) | | |
| Proportion Difference using Exact Method (95% CI) | 0.08 (-0.63, 0.76) | | |
| >=4 units transfused in the last 8 weeks | 1 ( 33.3%) | 1 ( 25.0%) | 2 ( 28.6%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 1 ( 33.3%) | 1 ( 25.0%) | 2 ( 28.6%) |
| Last Participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Other | 0 | 0 | 0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019

Table 2.4002: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 5 | | | |
| Transfusion Dependence No and Platelet Count >= 100X10E9/L and <= 200X10E9/L | 14 | 16 | 30 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Non-Dependent, n(%) | 12 ( 85.7%) | 8 ( 50.0%) | 20 ( 66.7%) |
| Transfusion Requiring, n(%) | 1 ( 7.1%) | 4 ( 25.0%) | 5 ( 16.7%) |
| Transfusion Independent, n(%) | 11 ( 78.6%) | 4 ( 25.0%) | 15 ( 50.0%) |
| Dependent, n(%) | 2 ( 14.3%) | 8 ( 50.0%) | 10 ( 33.3%) |
| 95% Exact CI | 0.0178, 0.4281 | 0.2465, 0.7535 | 0.1729, 0.5281 |
| Proportion Difference (95% CI) | -0.36 (-0.68, -0.03) | | |
| Proportion Difference using Exact Method (95% CI) | -0.36 (-0.66, -0.01) | | |
| >=4 units transfused in the last 8 weeks | 0 | 3 ( 18.8%) | 3 ( 10.0%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 2 ( 14.3%) | 7 ( 43.8%) | 9 ( 30.0%) |
| Last Participation date < Day 162 in DB phase | 0 | 0 | 0 |
| Other | 0 | 1 ( 6.3%) | 1 ( 3.3%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019

Table 2.4002: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| RBC Transfusion Dependent Rate at Week 12 | | | |
| Non-Dependent, n(%) | 14 (100.0%) | 4 ( 25.0%) | 18 ( 60.0%) |
| Transfusion Requiring, n(%) | 1 ( 7.1%) | 1 ( 6.3%) | 2 ( 6.7%) |
| Transfusion Independent, n(%) | 13 ( 92.9%) | 3 ( 18.8%) | 16 ( 53.3%) |
| Dependent, n(%) | 0 | 12 ( 75.0%) | 12 ( 40.0%) |
| 95% Exact CI | 0.0000, 0.2316 | 0.4762, 0.9273 | 0.2266, 0.5940 |
| Proportion Difference (95% CI) | -0.75 (-0.99, -0.51) | | |
| Proportion Difference using Exact Method (95% CI) | -0.75 (-0.93, -0.46) | | |
| >=4 units transfused in the last 8 weeks | 0 | 5 ( 31.3%) | 5 ( 16.7%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 0 | 10 ( 62.5%) | 10 ( 33.3%) |
| Last Participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Other | 0 | 3 ( 18.8%) | 3 ( 10.0%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019

Table 2.4002: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| Stratum 6 | | | |
| Transfusion Dependence No and Platelet Count > 200X10E9/L | 14 | 31 | 45 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Non-Dependent, n(%) | 11 ( 78.6%) | 16 ( 51.6%) | 27 ( 60.0%) |
| Transfusion Requiring, n(%) | 0 | 3 ( 9.7%) | 3 ( 6.7%) |
| Transfusion Independent, n(%) | 11 ( 78.6%) | 13 ( 41.9%) | 24 ( 53.3%) |
| Dependent, n(%) | 3 ( 21.4%) | 15 ( 48.4%) | 18 ( 40.0%) |
| 95% Exact CI | 0.0466, 0.5080 | 0.3015, 0.6694 | 0.2570, 0.5567 |
| Proportion Difference (95% CI) | -0.27 (-0.56, 0.02) | | |
| Proportion Difference using Exact Method (95% CI) | -0.27 (-0.56, 0.05) | | |
| >=4 units transfused in the last 8 weeks | 0 | 8 ( 25.8%) | 8 ( 17.8%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 1 ( 7.1%) | 13 ( 41.9%) | 14 ( 31.1%) |
| Last Participation date < Day 162 in DB phase | 2 ( 14.3%) | 0 | 2 ( 4.4%) |
| Other | 0 | 9 ( 29.0%) | 9 ( 20.0%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019

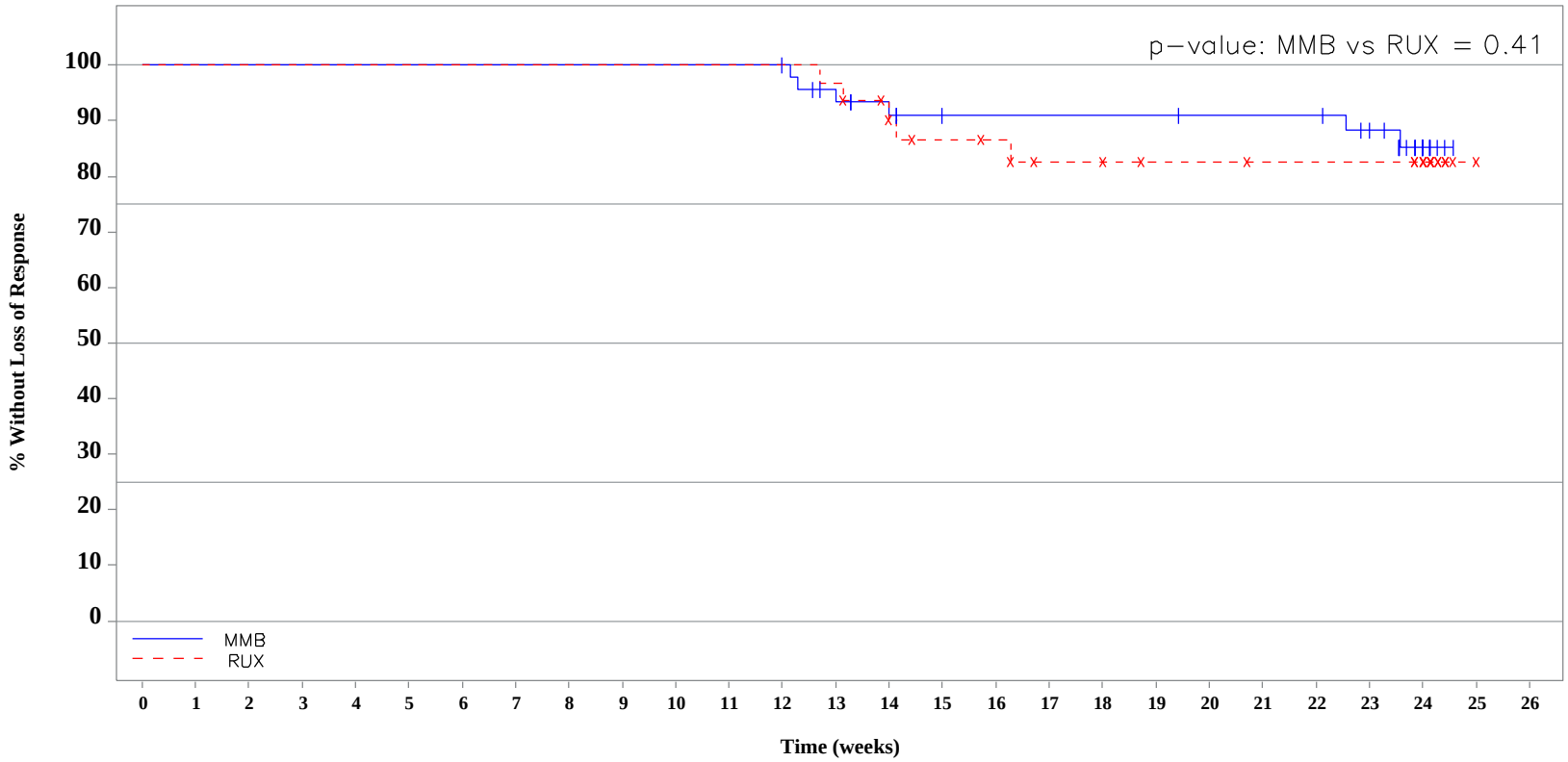
Table 2.4002: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Double-Blind Phase
PP-Anemic Analysis Set

| | MMB (N=68) | RUX (N=87) | Total (N=155) |
|---|---|---|---|
| RBC Transfusion Dependent Rate at Week 12 | | | |
| Non-Dependent, n(%) | 14 (100.0%) | 13 ( 41.9%) | 27 ( 60.0%) |
| Transfusion Requiring, n(%) | 3 ( 21.4%) | 1 ( 3.2%) | 4 ( 8.9%) |
| Transfusion Independent, n(%) | 11 ( 78.6%) | 12 ( 38.7%) | 23 ( 51.1%) |
| Dependent, n(%) | 0 | 18 ( 58.1%) | 18 ( 40.0%) |
| 95% Exact CI | 0.0000, 0.2316 | 0.3908, 0.7545 | 0.2570, 0.5567 |
| Proportion Difference (95% CI) | -0.58 (-0.78, -0.38) | | |
| Proportion Difference using Exact Method (95% CI) | -0.58 (-0.82, -0.28) | | |
| >=4 units transfused in the last 8 weeks | 0 | 7 ( 22.6%) | 7 ( 15.6%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 0 | 17 ( 54.8%) | 17 ( 37.8%) |
| Last Participation date < Day 78 in DB phase | 0 | 0 | 0 |
| Other | 0 | 7 ( 22.6%) | 7 ( 15.6%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel
RBC = Red Blood Cell

Data Extracted: CRF data: 01JUL2019

Figure 2.2401: Kaplan-Meier Plot of Duration of RBC Transfusion Independent Response
Double-blind Phase
ITT-Anemic Analysis Set with RBC Transfusion Independent Response




**N at Risk (Events)**

| | | | | | | | | | | | | | | | | | | | | | | | | | | |
|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|
| **MMB** | 47 (0) | 47 (0) | 47 (0) | 47 (0) | 47 (0) | 47 (0) | 47 (0) | 47 (0) | 47 (0) | 47 (0) | 47 (0) | 47 (0) | 47 (0) | 42 (3) | 39 (4) | 36 (4) | 35 (4) | 35 (4) | 35 (4) | 35 (4) | 34 (4) | 34 (4) | 34 (4) | 31 (5) | 19 (6) | 0 (6) | 0 (6) |
| **RUX** | 31 (0) | 31 (0) | 31 (0) | 31 (0) | 31 (0) | 31 (0) | 31 (0) | 31 (0) | 31 (0) | 31 (0) | 31 (0) | 31 (0) | 31 (0) | 30 (1) | 27 (3) | 23 (4) | 22 (4) | 19 (5) | 19 (5) | 17 (5) | 17 (5) | 16 (5) | 16 (5) | 16 (5) | 14 (5) | 1 (5) | 0 (5) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
Stratified two-sided p-value is from a log-rank test comparing treatment stratified by baseline TD (yes,no) and baseline platelet count (<100,100-200,>200 10^9/L).

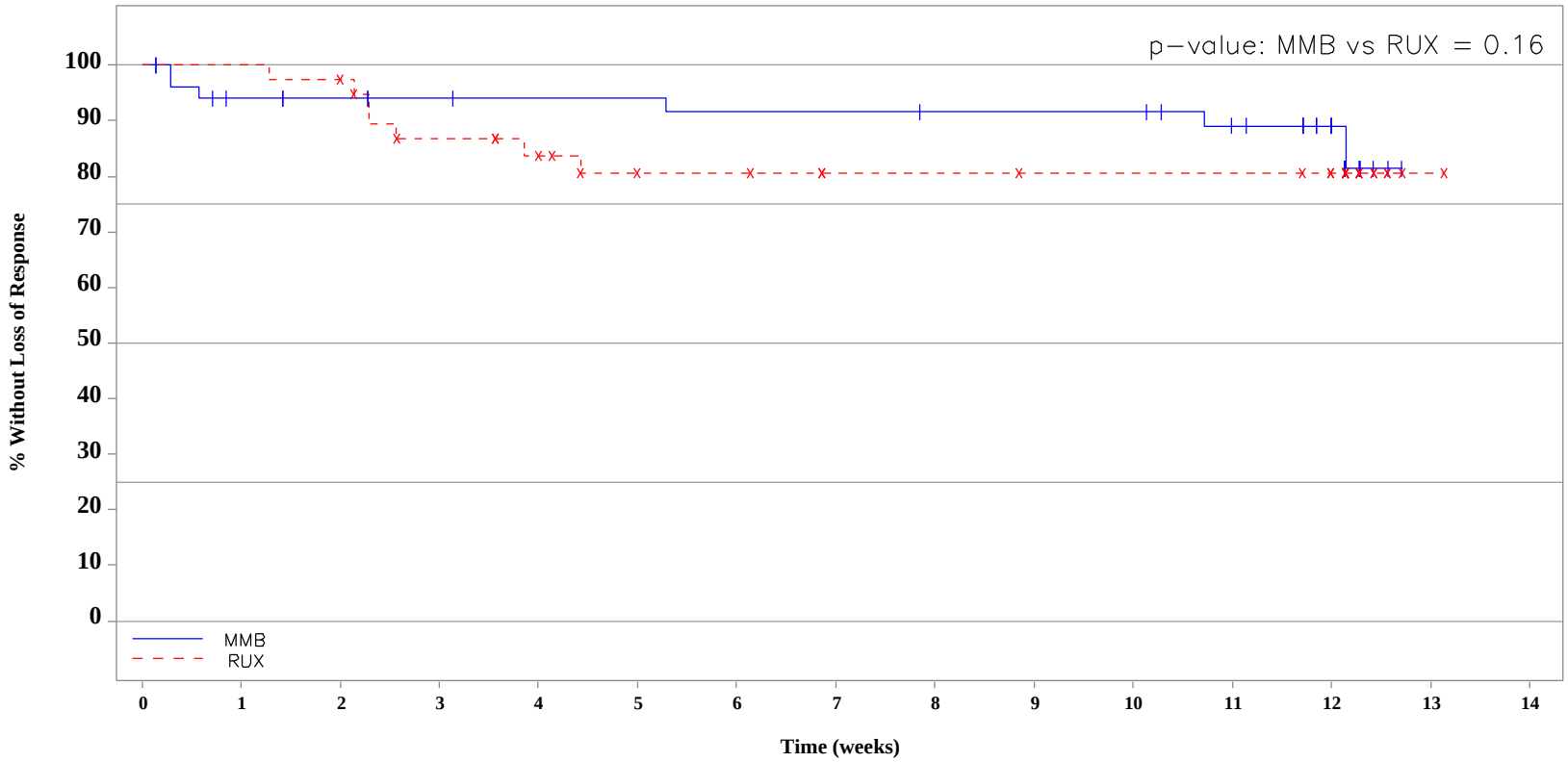
Data Extracted: CRF data: 01JUL2019
Source: g-dur-ti_Sep2023.sas V.03.05 Output file: g-dur-ti.pdf 27SEP2023:13:00

Table 2.3701: Analysis of Duration of RBC Transfusion Independent Response
Double-blind Phase
ITT-Anemic Analysis Set With RBC TI Response

| | MMB (N=47) | RUX (N=31) |
|---|---|---|
| Subjects with Event | | |
| Loss of RBC Transfusion Independent Response, n(%) | 6 ( 12.8%) | 5 ( 16.1%) |
| Censor | | |
| Subjects Censored, n(%) | 41 ( 87.2%) | 26 ( 83.9%) |
| No Loss of Response: Censored at Last Subject Visit Date at DB Phase | 41 (100.0%) | 26 (100.0%) |
| Kaplan-Meier Estimate of Duration of RBC Transfusion Independent Response (Weeks) | | |
| 25-percentile (95% CI) | NE (22.57, NE) | NE (14.00, NE) |
| Median (95% CI) | NE (NE, NE) | NE (NE, NE) |
| 75-percentile (95% CI) | NE (NE, NE) | NE (NE, NE) |
| Min, Max | 12.00, 24.57 | 12.71, 25.00 |
| Stratified Log-Rank Test p-value | 0.41 | |
| Adjusted Hazard Ratio (95% CI) | 0.71 (0.21, 2.45) | |
| Unstratified Log-Rank Test p-value | 0.69 | |
| Unadjusted Hazard Ratio (95% CI) | 0.78 (0.24, 2.58) | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
RBC transfusion independent response is defined as the absence of RBC transfusions and no hemoglobin level below 8 g/dL in the prior 12 weeks.
Stratified two-sided p-value is from a log-rank test comparing treatment stratified by baseline TD (yes,no) and baseline platelet count (<100,100-200,>200 10^9/L). Adjusted hazard ratio and 95% CI are from a Cox proportional hazards model with covariates of treatment, baseline TD (yes,no) and baseline platelet count (<100,100-200,>200 10^9/L). Unstratified two-sided p-value is from a log-rank test comparing treatment. Unadjusted hazard ratio and 95% CI are from a Cox proportional hazards model with a covariate of treatment.
P-value for interaction test is from a Cox proportional hazards model with covariates of treatment, subgroup, baseline TD (yes,no), baseline platelet count (<100,100-200,>200 10^9/L) and treatment by subgroup interaction. NE = Not estimable.
Data Extracted: CRF data: 01JUL2019
Source: t-dur-ti_Sep2023.sas V.03.05 Output file: t-dur-ti.pdf 26SEP2023:19:57

Figure 2.2701: Kaplan-Meier Plot of Duration of Any Type Transfusion Free Response
Double-blind Phase
ITT-Anemic Analysis Set With Any Type Transfusion Free Response



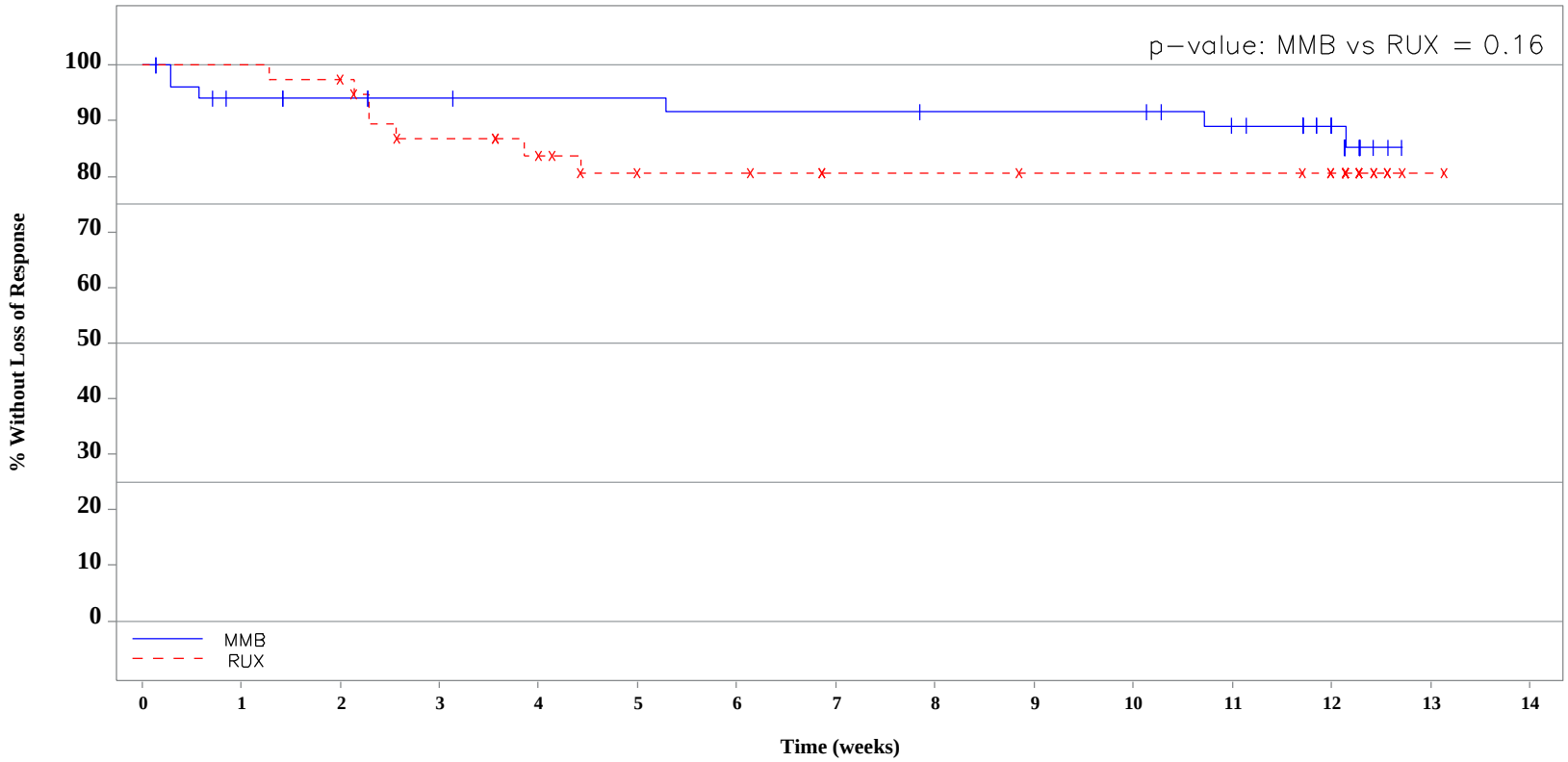

**N at Risk (Events)**

| | | | | | | | | | | | | | | | |
|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|
| **MMB** | 52 (0) | 45 (3) | 43 (3) | 41 (3) | 40 (3) | 40 (3) | 39 (4) | 39 (4) | 38 (4) | 38 (4) | 38 (4) | 35 (5) | 26 (5) | 0 (7) | 0 (7) |
| **RUX** | 39 (0) | 39 (0) | 38 (1) | 31 (5) | 28 (6) | 24 (7) | 23 (7) | 20 (7) | 20 (7) | 19 (7) | 19 (7) | 19 (7) | 18 (7) | 1 (7) | 0 (7) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
Stratified two-sided p-value is from a log-rank test comparing treatment stratified by baseline TD (yes,no) and baseline platelet count (<100,100-200,>200 10^9/L).

Data Extracted: CRF data: 01JUL2019
Source: g-dur-any-tfr_Sep2023.sas V.03.05 Output file: g-dur-any-tfr.pdf 27SEP2023:13:00

Figure 2.1801: Kaplan-Meier Plot of Duration of RBC Transfusion Free Response
Double-blind Phase
ITT-Anemic Analysis Set with RBC Transfusion Free Response




**N at Risk (Events)**

| | | | | | | | | | | | | | | | |
|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|
| **MMB** | 52 (0) | 45 (3) | 43 (3) | 41 (3) | 40 (3) | 40 (3) | 39 (4) | 39 (4) | 38 (4) | 38 (4) | 38 (4) | 35 (5) | 26 (5) | 0 (6) | 0 (6) |
| **RUX** | 39 (0) | 39 (0) | 38 (1) | 31 (5) | 28 (6) | 24 (7) | 23 (7) | 20 (7) | 20 (7) | 19 (7) | 19 (7) | 19 (7) | 18 (7) | 1 (7) | 0 (7) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
Stratified two-sided p-value is from a log-rank test comparing treatment stratified by baseline TD (yes,no) and baseline platelet count (<100,100-200,>200 10^9/L).

Data Extracted: CRF data: 01JUL2019
Source: g-dur-rbc-tfr_Sep2023.sas V.03.05 Output file: g-dur-rbc-tfr.pdf 27SEP2023:16:08

Table 2.4401: Analysis of Duration of Any Type Transfusion Free Response
Double-blind Phase
ITT-Anemic Analysis Set With Any Type Transfusion Free Response

| | MMB (N=52) | RUX (N=39) |
|---|---|---|
| Subjects with Event | | |
| Loss of Any Type Transfusion Free Response, n(%) | 7 ( 13.5%) | 7 ( 17.9%) |
| Censor | | |
| Subjects Censored, n(%) | 45 ( 86.5%) | 32 ( 82.1%) |
| No Loss of Response: Censored at Last Subject Visit Date at DB Phase | 45 (100.0%) | 32 (100.0%) |
| Kaplan-Meier Estimate of Duration of Any Type Transfusion Free Response (Weeks) | | |
| 25-percentile (95% CI) | NE (12.14, NE) | NE (2.29, NE) |
| Median (95% CI) | NE (NE, NE) | NE (NE, NE) |
| 75-percentile (95% CI) | NE (NE, NE) | NE (NE, NE) |
| Min, Max | 0.14, 12.71 | 1.29, 13.14 |
| Stratified Log-Rank Test p-value | 0.16 | |
| Adjusted Hazard Ratio (95% CI) | 0.59 (0.20, 1.75) | |
| Unstratified Log-Rank Test p-value | 0.56 | |
| Unadjusted Hazard Ratio (95% CI) | 0.74 (0.26, 2.11) | |

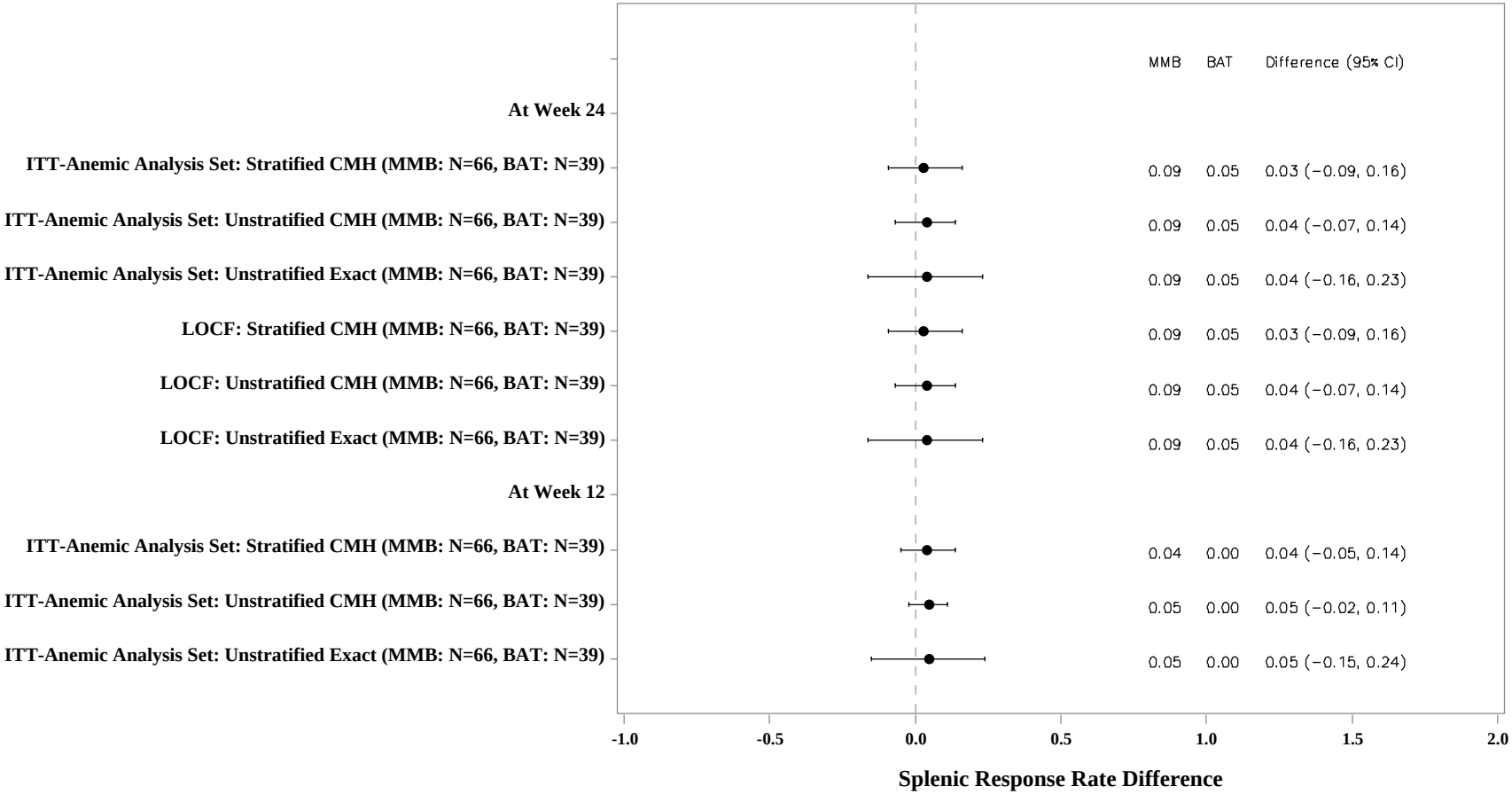
ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
Any type transfusion free response is defined as the absence of transfusions of any type in the prior 12 weeks.
Stratified two-sided p-value is from a log-rank test comparing treatment stratified by baseline TD (yes,no) and baseline platelet count (<100,100-200,>200 10^9/L).
Adjusted hazard ratio and 95% CI are from a Cox proportional hazards model with covariates of treatment, baseline TD (yes,no) and baseline platelet count (<100,100-200,>200 10^9/L). Unstratified two-sided p-value is from a log-rank test comparing treatment.
Unadjusted hazard ratio and 95% CI are from a Cox proportional hazards model with a covariate of treatment.
NE = Not estimable.
Data Extracted: CRF data: 01JUL2019
Source: t-dur-any-tfr_Sep2023.sas V.03.05 Output file: t-dur-any-tfr.pdf 26SEP2023:19:56

Table 2.2601: Analysis of Duration of RBC Transfusion Free Response
Double-blind Phase
ITT-Anemic Analysis Set With RBC TF Response

| | MMB (N=52) | RUX (N=39) |
|---|---|---|
| **Subjects with Event** | | |
| Loss of RBC Transfusion Free Response, n(%) | 6 ( 11.5%) | 7 ( 17.9%) |
| **Censor** | | |
| Subjects Censored, n(%) | 46 ( 88.5%) | 32 ( 82.1%) |
| No Loss of Response: Censored at Last Subject Visit Date at DB Phase | 46 (100.0%) | 32 (100.0%) |
| **Kaplan-Meier Estimate of Duration of RBC Transfusion Free Response (Weeks)** | | |
| 25-percentile (95% CI) | NE (12.14, NE) | NE (2.29, NE) |
| Median (95% CI) | NE (NE, NE) | NE (NE, NE) |
| 75-percentile (95% CI) | NE (NE, NE) | NE (NE, NE) |
| Min, Max | 0.14, 12.71 | 1.29, 13.14 |
| **Stratified Log-Rank Test p-value** | 0.16 | |
| **Adjusted Hazard Ratio (95% CI)** | 0.49 (0.16, 1.52) | |
| **Unstratified Log-Rank Test p-value** | 0.41 | |
| **Unadjusted Hazard Ratio (95% CI)** | 0.63 (0.21, 1.89) | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
RBC transfusion free response is defined as the absence of RBC transfusions in the prior 12 weeks.
Stratified two-sided p-value is from a log-rank test comparing treatment stratified by baseline TD (yes,no) and baseline platelet count (<100,100-200,>200 10^9/L).
Adjusted hazard ratio and 95% CI are from a Cox proportional hazards model with covariates of treatment, baseline TD (yes,no) and baseline platelet count (<100,100-200,>200 10^9/L). Unstratified two-sided p-value is from a log-rank test comparing treatment.
Unadjusted hazard ratio and 95% CI are from a Cox proportional hazards model with a covariate of treatment.
NE = Not estimable.
Data Extracted: CRF data: 01JUL2019
Source: t-dur-rbc-tfr_Sep2023.sas V.03.05  Output file: t-dur-rbc-tfr.pdf 26SEP2023:19:57

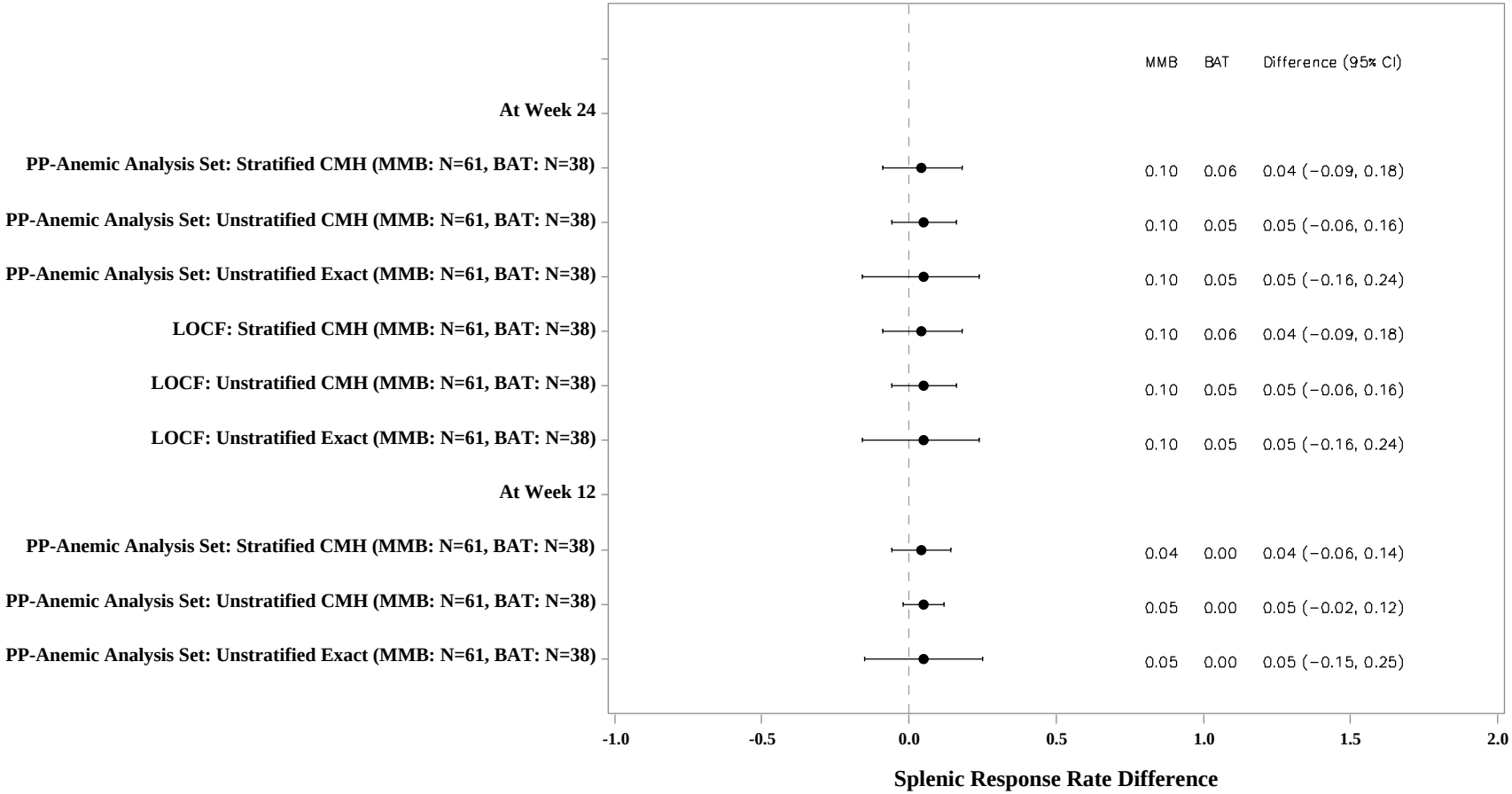
Figure 2.0201: Forest Plot of Primary and Sensitivity Analysis of Splenic Response Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set
All strata combined




ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
To the right of the reference line favors MMB, to the left favors BAT.
CMH = Cochran-Mantel-Haenszel; LOCF = Last Observation Carried Forward; CI= Confidence Interval.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/g-srr-forest.sas V.03.05 Output file: g-srr-forest.pdf 25AUG2023:13:36

Figure 2.0202: Forest Plot of Primary and Sensitivity Analysis of Splenic Response Rate at Week 24 and Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set
All strata combined




PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
To the right of the reference line favors MMB, to the left favors BAT.
CMH = Cochran-Mantel-Haenszel; LOCF = Last Observation Carried Forward; PP=Per-Protocol; CI= Confidence Interval.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/g-srr-forest.sas V.03.05 Output file: g-srr-forest-pp.pdf 25AUG2023:13:36

Table 2.0701: Sensitivity Analysis of Splenic Response Rate at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **All Strata Combined** | | | |
| **Splenic Response Rate at Week 24** | | | |
| Responder, n(%) | 6 ( 9.1%) | 2 ( 5.1%) | 8 ( 7.6%) |
| 95% Exact CI | 0.0341, 0.1874 | 0.0063, 0.1732 | 0.0335, 0.1446 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.03 (-0.09, 0.16) | | |
| p-value | 0.59 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.04 (-0.07, 0.14) | | |
| p-value | 0.46 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.04 (-0.16, 0.23) | | |
| p-value | 0.71 | | |
| Non-Responder, n(%) | 60 ( 90.9%) | 37 ( 94.9%) | 97 ( 92.4%) |
| Baseline spleen volume not available | 0 | 0 | 0 |
| Spleen volume at Week 24 not available | 12 ( 18.2%) | 8 ( 20.5%) | 20 ( 19.0%) |
| >0% spleen volume increase at Week 24 | 24 ( 36.4%) | 15 ( 38.5%) | 39 ( 37.1%) |
| <35% spleen volume reduction at Week 24 | 48 ( 72.7%) | 29 ( 74.4%) | 77 ( 73.3%) |
| Last participation date < Day 141 in RT Phase | 15 ( 22.7%) | 7 ( 17.9%) | 22 ( 21.0%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward.
CMH = Cochran-Mantel-Haenszel.

Data Extracted: CRF data: 25JUN2019

Table 2.0701: Sensitivity Analysis of Splenic Response Rate at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Splenic Response Rate at Any Timepoint in RT Phase | | | |
| Responder, n(%) | 6 ( 9.1%) | 2 ( 5.1%) | 8 ( 7.6%) |
| 95% Exact CI | 0.0341, 0.1874 | 0.0063, 0.1732 | 0.0335, 0.1446 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward.
CMH = Cochran-Mantel-Haenszel.

Data Extracted: CRF data: 25JUN2019

Table 2.0701: Sensitivity Analysis of Splenic Response Rate at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Baseline TSS <18 | 35 | 14 | 49 |
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 4 ( 11.4%) | 1 ( 7.1%) | 5 ( 10.2%) |
| 95% Exact CI | 0.0320, 0.2674 | 0.0018, 0.3387 | 0.0340, 0.2223 |
| Proportion Difference (95% CI) | 0.04 (-0.15, 0.24) | | |
| Proportion Difference using Exact Method (95% CI) | 0.04 (-0.27, 0.35) | | |
| Non-Responder, n(%) | 31 ( 88.6%) | 13 ( 92.9%) | 44 ( 89.8%) |
| Baseline spleen volume not available | 0 | 0 | 0 |
| Spleen volume at Week 24 not available | 4 ( 11.4%) | 2 ( 14.3%) | 6 ( 12.2%) |
| >0% spleen volume increase at Week 24 | 16 ( 45.7%) | 6 ( 42.9%) | 22 ( 44.9%) |
| <35% spleen volume reduction at Week 24 | 27 ( 77.1%) | 11 ( 78.6%) | 38 ( 77.6%) |
| Last participation date < Day 141 in RT Phase | 5 ( 14.3%) | 2 ( 14.3%) | 7 ( 14.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward.
CMH = Cochran-Mantel-Haenszel.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-srr24.sas V.03.05 Output file: t-sen-locf-srr24.pdf 24AUG2023:16:54

Table 2.0701: Sensitivity Analysis of Splenic Response Rate at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Splenic Response Rate at Any Timepoint in RT Phase | | | |
| Responder, n(%) | 4 ( 11.4%) | 1 ( 7.1%) | 5 ( 10.2%) |
| 95% Exact CI | 0.0320, 0.2674 | 0.0018, 0.3387 | 0.0340, 0.2223 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward.
CMH = Cochran-Mantel-Haenszel.

Data Extracted: CRF data: 25JUN2019

Table 2.0701: Sensitivity Analysis of Splenic Response Rate at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Baseline TSS >=18 | 17 | 11 | 28 |
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 1 ( 5.9%) | 0 | 1 ( 3.6%) |
| 95% Exact CI | 0.0015, 0.2869 | 0.0000, 0.2849 | 0.0009, 0.1835 |
| Proportion Difference (95% CI) | 0.06 (-0.12, 0.24) | | |
| Proportion Difference using Exact Method (95% CI) | 0.06 (-0.31, 0.42) | | |
| Non-Responder, n(%) | 16 ( 94.1%) | 11 (100.0%) | 27 ( 96.4%) |
| Baseline spleen volume not available | 0 | 0 | 0 |
| Spleen volume at Week 24 not available | 3 ( 17.6%) | 3 ( 27.3%) | 6 ( 21.4%) |
| >0% spleen volume increase at Week 24 | 4 ( 23.5%) | 4 ( 36.4%) | 8 ( 28.6%) |
| <35% spleen volume reduction at Week 24 | 13 ( 76.5%) | 8 ( 72.7%) | 21 ( 75.0%) |
| Last participation date < Day 141 in RT Phase | 4 ( 23.5%) | 2 ( 18.2%) | 6 ( 21.4%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward.
CMH = Cochran-Mantel-Haenszel.

Data Extracted: CRF data: 25JUN2019

Table 2.0701: Sensitivity Analysis of Splenic Response Rate at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Splenic Response Rate at Any Timepoint in RT Phase | | | |
| Responder, n(%) | 1 ( 5.9%) | 0 | 1 ( 3.6%) |
| 95% Exact CI | 0.0015, 0.2869 | 0.0000, 0.2849 | 0.0009, 0.1835 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward.
CMH = Cochran-Mantel-Haenszel.

Data Extracted: CRF data: 25JUN2019

Table 2.0701: Sensitivity Analysis of Splenic Response Rate at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence No and Baseline TSS <18 | 7 | 6 | 13 |
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 0 | 1 ( 16.7%) | 1 ( 7.7%) |
| 95% Exact CI | 0.0000, 0.4096 | 0.0042, 0.6412 | 0.0019, 0.3603 |
| Proportion Difference (95% CI) | -0.17 (-0.58, 0.24) | | |
| Proportion Difference using Exact Method (95% CI) | -0.17 (-0.64, 0.37) | | |
| Non-Responder, n(%) | 7 (100.0%) | 5 ( 83.3%) | 12 ( 92.3%) |
| Baseline spleen volume not available | 0 | 0 | 0 |
| Spleen volume at Week 24 not available | 3 ( 42.9%) | 1 ( 16.7%) | 4 ( 30.8%) |
| >0% spleen volume increase at Week 24 | 2 ( 28.6%) | 2 ( 33.3%) | 4 ( 30.8%) |
| <35% spleen volume reduction at Week 24 | 4 ( 57.1%) | 4 ( 66.7%) | 8 ( 61.5%) |
| Last participation date < Day 141 in RT Phase | 3 ( 42.9%) | 1 ( 16.7%) | 4 ( 30.8%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward.
CMH = Cochran-Mantel-Haenszel.

Data Extracted: CRF data: 25JUN2019

Table 2.0701: Sensitivity Analysis of Splenic Response Rate at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Splenic Response Rate at Any Timepoint in RT Phase | | | |
| Responder, n(%) | 0 | 1 ( 16.7%) | 1 ( 7.7%) |
| 95% Exact CI | 0.0000, 0.4096 | 0.0042, 0.6412 | 0.0019, 0.3603 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward.
CMH = Cochran-Mantel-Haenszel.

Data Extracted: CRF data: 25JUN2019

Table 2.0701: Sensitivity Analysis of Splenic Response Rate at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Baseline TSS >=18 | 7 | 8 | 15 |
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 1 ( 14.3%) | 0 | 1 ( 6.7%) |
| 95% Exact CI | 0.0036, 0.5787 | 0.0000, 0.3694 | 0.0017, 0.3195 |
| Proportion Difference (95% CI) | 0.14 (-0.21, 0.50) | | |
| Proportion Difference using Exact Method (95% CI) | 0.14 (-0.36, 0.58) | | |
| Non-Responder, n(%) | 6 ( 85.7%) | 8 (100.0%) | 14 ( 93.3%) |
| Baseline spleen volume not available | 0 | 0 | 0 |
| Spleen volume at Week 24 not available | 2 ( 28.6%) | 2 ( 25.0%) | 4 ( 26.7%) |
| >0% spleen volume increase at Week 24 | 2 ( 28.6%) | 3 ( 37.5%) | 5 ( 33.3%) |
| <35% spleen volume reduction at Week 24 | 4 ( 57.1%) | 6 ( 75.0%) | 10 ( 66.7%) |
| Last participation date < Day 141 in RT Phase | 3 ( 42.9%) | 2 ( 25.0%) | 5 ( 33.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward.
CMH = Cochran-Mantel-Haenszel.

Data Extracted: CRF data: 25JUN2019

Table 2.0701: Sensitivity Analysis of Splenic Response Rate at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Splenic Response Rate at Any Timepoint in RT Phase | | | |
| Responder, n(%) | 1 ( 14.3%) | 0 | 1 ( 6.7%) |
| 95% Exact CI | 0.0036, 0.5787 | 0.0000, 0.3694 | 0.0017, 0.3195 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
LOCF = Last Observation Carried Forward.
CMH = Cochran-Mantel-Haenszel.

Data Extracted: CRF data: 25JUN2019

Table 2.0702: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **All Strata Combined** | | | |
| **Splenic Response Rate at Week 24** | | | |
| Responder, n(%) | 6 ( 9.8%) | 2 ( 5.3%) | 8 ( 8.1%) |
| 95% Exact CI | 0.0370, 0.2019 | 0.0064, 0.1775 | 0.0355, 0.1530 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.04 (-0.09, 0.18) | | |
| p-value | 0.53 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.05 (-0.06, 0.16) | | |
| p-value | 0.42 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.05 (-0.16, 0.24) | | |
| p-value | 0.71 | | |
| Non-Responder, n(%) | 55 ( 90.2%) | 36 ( 94.7%) | 91 ( 91.9%) |
| Spleen volume at Week 24 not available | 19 ( 31.1%) | 9 ( 23.7%) | 28 ( 28.3%) |
| >0% spleen volume increase at Week 24 | 18 ( 29.5%) | 14 ( 36.8%) | 32 ( 32.3%) |
| <35% spleen volume reduction at Week 24 | 36 ( 59.0%) | 27 ( 71.1%) | 63 ( 63.6%) |
| Last participation date < Day 141 in RT Phase | 13 ( 21.3%) | 7 ( 18.4%) | 20 ( 20.2%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol.

Data Extracted: CRF data: 25JUN2019

Table 2.0702: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **Splenic Response Rate at Week 12** | | | |
| Responder, n(%) | 3 ( 4.9%) | 0 | 3 ( 3.0%) |
| 95% Exact CI | 0.0103, 0.1371 | 0.0000, 0.0925 | 0.0063, 0.0860 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.04 (-0.06, 0.14) | | |
| p-value | 0.39 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.05 (-0.02, 0.12) | | |
| p-value | 0.16 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.05 (-0.15, 0.25) | | |
| p-value | 0.28 | | |
| Non-Responder, n(%) | 58 ( 95.1%) | 38 (100.0%) | 96 ( 97.0%) |
| Spleen volume at Week 12 not available | 11 ( 18.0%) | 7 ( 18.4%) | 18 ( 18.2%) |
| >0% spleen volume increase at Week 12 | 21 ( 34.4%) | 16 ( 42.1%) | 37 ( 37.4%) |
| <35% spleen volume reduction at Week 12 | 47 ( 77.0%) | 31 ( 81.6%) | 78 ( 78.8%) |
| Last participation date < Day 57 in RT Phase | 4 ( 6.6%) | 4 ( 10.5%) | 8 ( 8.1%) |
| **Splenic Response Rate at Any Timepoint in RT Phase** | | | |
| Responder, n(%) | 6 ( 9.8%) | 2 ( 5.3%) | 8 ( 8.1%) |
| 95% Exact CI | 0.0370, 0.2019 | 0.0064, 0.1775 | 0.0355, 0.1530 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-srr24.sas V.03.05 Output file: t-sen-ppt-srr24.pdf 24AUG2023:16:53

Table 2.0702: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Baseline TSS <18 | 32 | 14 | 46 |
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 4 ( 12.5%) | 1 ( 7.1%) | 5 ( 10.9%) |
| 95% Exact CI | 0.0351, 0.2899 | 0.0018, 0.3387 | 0.0362, 0.2357 |
| Proportion Difference (95% CI) | 0.05 (-0.15, 0.26) | | |
| Proportion Difference using Exact Method (95% CI) | 0.05 (-0.26, 0.36) | | |
| Non-Responder, n(%) | 28 ( 87.5%) | 13 ( 92.9%) | 41 ( 89.1%) |
| Spleen volume at Week 24 not available | 8 ( 25.0%) | 3 ( 21.4%) | 11 ( 23.9%) |
| >0% spleen volume increase at Week 24 | 12 ( 37.5%) | 6 ( 42.9%) | 18 ( 39.1%) |
| <35% spleen volume reduction at Week 24 | 20 ( 62.5%) | 10 ( 71.4%) | 30 ( 65.2%) |
| Last participation date < Day 141 in RT Phase | 5 ( 15.6%) | 2 ( 14.3%) | 7 ( 15.2%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-srr24.sas V.03.05 Output file: t-sen-ppt-srr24.pdf 24AUG2023:16:53

Table 2.0702: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **Splenic Response Rate at Week 12** | | | |
| Responder, n(%) | 2 ( 6.3%) | 0 | 2 ( 4.3%) |
| 95% Exact CI | 0.0077, 0.2081 | 0.0000, 0.2316 | 0.0053, 0.1484 |
| Proportion Difference (95% CI) | 0.06 (-0.07, 0.20) | | |
| Proportion Difference using Exact Method (95% CI) | 0.06 (-0.25, 0.37) | | |
| Non-Responder, n(%) | 30 ( 93.8%) | 14 (100.0%) | 44 ( 95.7%) |
| Spleen volume at Week 12 not available | 4 ( 12.5%) | 2 ( 14.3%) | 6 ( 13.0%) |
| >0% spleen volume increase at Week 12 | 13 ( 40.6%) | 7 ( 50.0%) | 20 ( 43.5%) |
| <35% spleen volume reduction at Week 12 | 26 ( 81.3%) | 12 ( 85.7%) | 38 ( 82.6%) |
| Last participation date < Day 57 in RT Phase | 0 | 2 ( 14.3%) | 2 ( 4.3%) |
| **Splenic Response Rate at Any Timepoint in RT Phase** | | | |
| Responder, n(%) | 4 ( 12.5%) | 1 ( 7.1%) | 5 ( 10.9%) |
| 95% Exact CI | 0.0351, 0.2899 | 0.0018, 0.3387 | 0.0362, 0.2357 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol.

Data Extracted: CRF data: 25JUN2019

Table 2.0702: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Baseline TSS >=18 | 17 | 10 | 27 |
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 1 ( 5.9%) | 0 | 1 ( 3.7%) |
| 95% Exact CI | 0.0015, 0.2869 | 0.0000, 0.3085 | 0.0009, 0.1897 |
| Proportion Difference (95% CI) | 0.06 (-0.13, 0.25) | | |
| Proportion Difference using Exact Method (95% CI) | 0.06 (-0.32, 0.43) | | |
| Non-Responder, n(%) | 16 ( 94.1%) | 10 (100.0%) | 26 ( 96.3%) |
| Spleen volume at Week 24 not available | 6 ( 35.3%) | 3 ( 30.0%) | 9 ( 33.3%) |
| >0% spleen volume increase at Week 24 | 3 ( 17.6%) | 3 ( 30.0%) | 6 ( 22.2%) |
| <35% spleen volume reduction at Week 24 | 10 ( 58.8%) | 7 ( 70.0%) | 17 ( 63.0%) |
| Last participation date < Day 141 in RT Phase | 4 ( 23.5%) | 2 ( 20.0%) | 6 ( 22.2%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol.

Data Extracted: CRF data: 25JUN2019

Table 2.0702: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **Splenic Response Rate at Week 12** | | | |
| Responder, n(%) | 1 ( 5.9%) | 0 | 1 ( 3.7%) |
| 95% Exact CI | 0.0015, 0.2869 | 0.0000, 0.3085 | 0.0009, 0.1897 |
| Proportion Difference (95% CI) | 0.06 (-0.13, 0.25) | | |
| Proportion Difference using Exact Method (95% CI) | 0.06 (-0.32, 0.43) | | |
| Non-Responder, n(%) | 16 ( 94.1%) | 10 (100.0%) | 26 ( 96.3%) |
| Spleen volume at Week 12 not available | 4 ( 23.5%) | 2 ( 20.0%) | 6 ( 22.2%) |
| >0% spleen volume increase at Week 12 | 5 ( 29.4%) | 3 ( 30.0%) | 8 ( 29.6%) |
| <35% spleen volume reduction at Week 12 | 12 ( 70.6%) | 8 ( 80.0%) | 20 ( 74.1%) |
| Last participation date < Day 57 in RT Phase | 2 ( 11.8%) | 2 ( 20.0%) | 4 ( 14.8%) |
| **Splenic Response Rate at Any Timepoint in RT Phase** | | | |
| Responder, n(%) | 1 ( 5.9%) | 0 | 1 ( 3.7%) |
| 95% Exact CI | 0.0015, 0.2869 | 0.0000, 0.3085 | 0.0009, 0.1897 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol.

Data Extracted: CRF data: 25JUN2019

Table 2.0702: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence No and Baseline TSS <18 | 7 | 6 | 13 |
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 0 | 1 ( 16.7%) | 1 ( 7.7%) |
| 95% Exact CI | 0.0000, 0.4096 | 0.0042, 0.6412 | 0.0019, 0.3603 |
| Proportion Difference (95% CI) | -0.17 (-0.58, 0.24) | | |
| Proportion Difference using Exact Method (95% CI) | -0.17 (-0.64, 0.37) | | |
| Non-Responder, n(%) | 7 (100.0%) | 5 ( 83.3%) | 12 ( 92.3%) |
| Spleen volume at Week 24 not available | 4 ( 57.1%) | 1 ( 16.7%) | 5 ( 38.5%) |
| >0% spleen volume increase at Week 24 | 1 ( 14.3%) | 2 ( 33.3%) | 3 ( 23.1%) |
| <35% spleen volume reduction at Week 24 | 3 ( 42.9%) | 4 ( 66.7%) | 7 ( 53.8%) |
| Last participation date < Day 141 in RT Phase | 3 ( 42.9%) | 1 ( 16.7%) | 4 ( 30.8%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol.

Data Extracted: CRF data: 25JUN2019

Table 2.0702: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 0 | 0 | 0 |
| 95% Exact CI | 0.0000, 0.4096 | 0.0000, 0.4593 | 0.0000, 0.2471 |
| Proportion Difference (95% CI) | 0.00 (-0.30, 0.30) | | |
| Proportion Difference using Exact Method (95% CI) | | | |
| Non-Responder, n(%) | 7 (100.0%) | 6 (100.0%) | 13 (100.0%) |
| Spleen volume at Week 12 not available | 3 ( 42.9%) | 0 | 3 ( 23.1%) |
| >0% spleen volume increase at Week 12 | 2 ( 28.6%) | 3 ( 50.0%) | 5 ( 38.5%) |
| <35% spleen volume reduction at Week 12 | 4 ( 57.1%) | 6 (100.0%) | 10 ( 76.9%) |
| Last participation date < Day 57 in RT Phase | 2 ( 28.6%) | 0 | 2 ( 15.4%) |
| Splenic Response Rate at Any Timepoint in RT Phase | | | |
| Responder, n(%) | 0 | 1 ( 16.7%) | 1 ( 7.7%) |
| 95% Exact CI | 0.0000, 0.4096 | 0.0042, 0.6412 | 0.0019, 0.3603 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol.

Data Extracted: CRF data: 25JUN2019

Table 2.0702: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Baseline TSS >=18 | 5 | 8 | 13 |
| Splenic Response Rate at Week 24 | | | |
| Responder, n(%) | 1 ( 20.0%) | 0 | 1 ( 7.7%) |
| 95% Exact CI | 0.0051, 0.7164 | 0.0000, 0.3694 | 0.0019, 0.3603 |
| Proportion Difference (95% CI) | 0.20 (-0.26, 0.66) | | |
| Proportion Difference using Exact Method (95% CI) | 0.20 (-0.35, 0.72) | | |
| Non-Responder, n(%) | 4 ( 80.0%) | 8 (100.0%) | 12 ( 92.3%) |
| Spleen volume at Week 24 not available | 1 ( 20.0%) | 2 ( 25.0%) | 3 ( 23.1%) |
| >0% spleen volume increase at Week 24 | 2 ( 40.0%) | 3 ( 37.5%) | 5 ( 38.5%) |
| <35% spleen volume reduction at Week 24 | 3 ( 60.0%) | 6 ( 75.0%) | 9 ( 69.2%) |
| Last participation date < Day 141 in RT Phase | 1 ( 20.0%) | 2 ( 25.0%) | 3 ( 23.1%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol.

Data Extracted: CRF data: 25JUN2019

Table 2.0702: Sensitivity Analysis of Splenic Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 0 | 0 | 0 |
| 95% Exact CI | 0.0000, 0.5218 | 0.0000, 0.3694 | 0.0000, 0.2471 |
| Proportion Difference (95% CI) | 0.00 (-0.32, 0.32) | | |
| Proportion Difference using Exact Method (95% CI) | | | |
| Non-Responder, n(%) | 5 (100.0%) | 8 (100.0%) | 13 (100.0%) |
| Spleen volume at Week 12 not available | 0 | 3 ( 37.5%) | 3 ( 23.1%) |
| >0% spleen volume increase at Week 12 | 1 ( 20.0%) | 3 ( 37.5%) | 4 ( 30.8%) |
| <35% spleen volume reduction at Week 12 | 5 (100.0%) | 5 ( 62.5%) | 10 ( 76.9%) |
| Last participation date < Day 57 in RT Phase | 0 | 0 | 0 |
| Splenic Response Rate at Any Timepoint in RT Phase | | | |
| Responder, n(%) | 1 ( 20.0%) | 0 | 1 ( 7.7%) |
| 95% Exact CI | 0.0051, 0.7164 | 0.0000, 0.3694 | 0.0019, 0.3603 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel; PP=Per-Protocol.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-srr24.sas V.03.05 Output file: t-sen-ppt-srr24.pdf 24AUG2023:16:53

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **All Strata Combined** | | | |
| **Splenic Response Rate at Week 12** | | | |
| Responder, n(%) | 3 ( 4.5%) | 0 | 3 ( 2.9%) |
| 95% Exact CI | 0.0095, 0.1271 | 0.0000, 0.0903 | 0.0059, 0.0812 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.04 (-0.05, 0.14) | | |
| p-value | 0.40 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.05 (-0.02, 0.11) | | |
| p-value | 0.17 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.05 (-0.15, 0.24) | | |
| p-value | 0.29 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 0.24 (0.01, 4.51) | | |
| p-value [1] | 0.34 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 0.23 (0.01, 4.57) | | |
| p-value [1] | 0.33 | | |
| Unadjusted Absolute Risk Difference (95% CI) | 0.04 (-0.02, 0.10) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | NE | | |
| p-value [2] | NE | | |
| Non-Responder, n(%) | 63 ( 95.5%) | 39 (100.0%) | 102 ( 97.1%) |
| Baseline spleen volume not available | 0 | 0 | 0 |
| Spleen volume at Week 12 not available | 13 ( 19.7%) | 8 ( 20.5%) | 21 ( 20.0%) |
| >0% spleen volume increase at Week 12 | 24 ( 36.4%) | 16 ( 41.0%) | 40 ( 38.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| <35% spleen volume reduction at Week 12 | 50 ( 75.8%) | 31 ( 79.5%) | 81 ( 77.1%) |
| Last participation date < Day 57 in RT phase | 5 ( 7.6%) | 4 ( 10.3%) | 9 ( 8.6%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **Splenic Response Rate at Week 24** | | | |
| Responder, n(%) | 6 ( 9.1%) | 2 ( 5.1%) | 8 ( 7.6%) |
| 95% Exact CI | 0.0341, 0.1874 | 0.0063, 0.1732 | 0.0335, 0.1446 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.03 (-0.09, 0.16) | | |
| p-value | 0.59 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.04 (-0.07, 0.14) | | |
| p-value | 0.46 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.04 (-0.16, 0.23) | | |
| p-value | 0.71 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 0.56 (0.12, 2.66) | | |
| p-value [1] | 0.47 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 0.54 (0.10, 2.82) | | |
| p-value [1] | 0.47 | | |
| Unadjusted Absolute Risk Difference (95% CI) | 0.04 (-0.06, 0.14) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | 0.60 (0.12, 2.93) | | |
| p-value [2] | 0.53 | | |
| Non-Responder, n(%) | 60 ( 90.9%) | 37 ( 94.9%) | 97 ( 92.4%) |
| Baseline spleen volume not available | 0 | 0 | 0 |
| Spleen volume at Week 24 not available | 22 ( 33.3%) | 9 ( 23.1%) | 31 ( 29.5%) |
| >0% spleen volume increase at Week 24 | 20 ( 30.3%) | 15 ( 38.5%) | 35 ( 33.3%) |
| <35% spleen volume reduction at Week 24 | 38 ( 57.6%) | 28 ( 71.8%) | 66 ( 62.9%) |
| Last participation date < Day 141 in RT phase | 15 ( 22.7%) | 7 ( 17.9%) | 22 ( 21.0%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **Splenic Response Rate at Any Timepoint in RT Phase** | | | |
| Responder, n(%) | 6 ( 9.1%) | 2 ( 5.1%) | 8 ( 7.6%) |
| 95% Exact CI | 0.0341, 0.1874 | 0.0063, 0.1732 | 0.0335, 0.1446 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Baseline TSS <18 | 35 | 14 | 49 |
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 2 ( 5.7%) | 0 | 2 ( 4.1%) |
| 95% Exact CI | 0.0070, 0.1916 | 0.0000, 0.2316 | 0.0050, 0.1398 |
| Proportion Difference (95% CI) | 0.06 (-0.07, 0.19) | | |
| Proportion Difference using Exact Method (95% CI) | 0.06 (-0.26, 0.36) | | |
| Non-Responder, n(%) | 33 ( 94.3%) | 14 (100.0%) | 47 ( 95.9%) |
| Baseline spleen volume not available | 0 | 0 | 0 |
| Spleen volume at Week 12 not available | 4 ( 11.4%) | 2 ( 14.3%) | 6 ( 12.2%) |
| >0% spleen volume increase at Week 12 | 16 ( 45.7%) | 7 ( 50.0%) | 23 ( 46.9%) |
| <35% spleen volume reduction at Week 12 | 29 ( 82.9%) | 12 ( 85.7%) | 41 ( 83.7%) |
| Last participation date < Day 57 in RT phase | 0 | 2 ( 14.3%) | 2 ( 4.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **Splenic Response Rate at Week 24** | | | |
| Responder, n(%) | 4 ( 11.4%) | 1 ( 7.1%) | 5 ( 10.2%) |
| 95% Exact CI | 0.0320, 0.2674 | 0.0018, 0.3387 | 0.0340, 0.2223 |
| Proportion Difference (95% CI) | 0.04 (-0.15, 0.24) | | |
| Proportion Difference using Exact Method (95% CI) | 0.04 (-0.27, 0.35) | | |
| Non-Responder, n(%) | 31 ( 88.6%) | 13 ( 92.9%) | 44 ( 89.8%) |
| Baseline spleen volume not available | 0 | 0 | 0 |
| Spleen volume at Week 24 not available | 9 ( 25.7%) | 3 ( 21.4%) | 12 ( 24.5%) |
| >0% spleen volume increase at Week 24 | 14 ( 40.0%) | 6 ( 42.9%) | 20 ( 40.8%) |
| <35% spleen volume reduction at Week 24 | 22 ( 62.9%) | 10 ( 71.4%) | 32 ( 65.3%) |
| Last participation date < Day 141 in RT phase | 5 ( 14.3%) | 2 ( 14.3%) | 7 ( 14.3%) |
| **Splenic Response Rate at Any Timepoint in RT Phase** | | | |
| Responder, n(%) | 4 ( 11.4%) | 1 ( 7.1%) | 5 ( 10.2%) |
| 95% Exact CI | 0.0320, 0.2674 | 0.0018, 0.3387 | 0.0340, 0.2223 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Baseline TSS >=18 | 17 | 11 | 28 |
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 1 ( 5.9%) | 0 | 1 ( 3.6%) |
| 95% Exact CI | 0.0015, 0.2869 | 0.0000, 0.2849 | 0.0009, 0.1835 |
| Proportion Difference (95% CI) | 0.06 (-0.12, 0.24) | | |
| Proportion Difference using Exact Method (95% CI) | 0.06 (-0.31, 0.42) | | |
| Non-Responder, n(%) | 16 ( 94.1%) | 11 (100.0%) | 27 ( 96.4%) |
| Baseline spleen volume not available | 0 | 0 | 0 |
| Spleen volume at Week 12 not available | 4 ( 23.5%) | 3 ( 27.3%) | 7 ( 25.0%) |
| >0% spleen volume increase at Week 12 | 5 ( 29.4%) | 3 ( 27.3%) | 8 ( 28.6%) |
| <35% spleen volume reduction at Week 12 | 12 ( 70.6%) | 8 ( 72.7%) | 20 ( 71.4%) |
| Last participation date < Day 57 in RT phase | 2 ( 11.8%) | 2 ( 18.2%) | 4 ( 14.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-srr24-gba.sas V.03.05 Output file: t-srr24-gba.pdf 24AUG2023:16:40

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **Splenic Response Rate at Week 24** | | | |
| Responder, n(%) | 1 ( 5.9%) | 0 | 1 ( 3.6%) |
| 95% Exact CI | 0.0015, 0.2869 | 0.0000, 0.2849 | 0.0009, 0.1835 |
| Proportion Difference (95% CI) | 0.06 (-0.12, 0.24) | | |
| Proportion Difference using Exact Method (95% CI) | 0.06 (-0.31, 0.42) | | |
| Non-Responder, n(%) | 16 ( 94.1%) | 11 (100.0%) | 27 ( 96.4%) |
| Baseline spleen volume not available | 0 | 0 | 0 |
| Spleen volume at Week 24 not available | 6 ( 35.3%) | 3 ( 27.3%) | 9 ( 32.1%) |
| >0% spleen volume increase at Week 24 | 3 ( 17.6%) | 4 ( 36.4%) | 7 ( 25.0%) |
| <35% spleen volume reduction at Week 24 | 10 ( 58.8%) | 8 ( 72.7%) | 18 ( 64.3%) |
| Last participation date < Day 141 in RT phase | 4 ( 23.5%) | 2 ( 18.2%) | 6 ( 21.4%) |
| **Splenic Response Rate at Any Timepoint in RT Phase** | | | |
| Responder, n(%) | 1 ( 5.9%) | 0 | 1 ( 3.6%) |
| 95% Exact CI | 0.0015, 0.2869 | 0.0000, 0.2849 | 0.0009, 0.1835 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence No and Baseline TSS <18 | 7 | 6 | 13 |
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 0 | 0 | 0 |
| 95% Exact CI | 0.0000, 0.4096 | 0.0000, 0.4593 | 0.0000, 0.2471 |
| Proportion Difference (95% CI) | 0.00 (-0.30, 0.30) | | |
| Proportion Difference using Exact Method (95% CI) | | | |
| Non-Responder, n(%) | 7 (100.0%) | 6 (100.0%) | 13 (100.0%) |
| Baseline spleen volume not available | 0 | 0 | 0 |
| Spleen volume at Week 12 not available | 3 ( 42.9%) | 0 | 3 ( 23.1%) |
| >0% spleen volume increase at Week 12 | 2 ( 28.6%) | 3 ( 50.0%) | 5 ( 38.5%) |
| <35% spleen volume reduction at Week 12 | 4 ( 57.1%) | 6 (100.0%) | 10 ( 76.9%) |
| Last participation date < Day 57 in RT phase | 2 ( 28.6%) | 0 | 2 ( 15.4%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **Splenic Response Rate at Week 24** | | | |
| Responder, n(%) | 0 | 1 ( 16.7%) | 1 ( 7.7%) |
| 95% Exact CI | 0.0000, 0.4096 | 0.0042, 0.6412 | 0.0019, 0.3603 |
| Proportion Difference (95% CI) | -0.17 (-0.58, 0.24) | | |
| Proportion Difference using Exact Method (95% CI) | -0.17 (-0.64, 0.37) | | |
| Non-Responder, n(%) | 7 (100.0%) | 5 ( 83.3%) | 12 ( 92.3%) |
| Baseline spleen volume not available | 0 | 0 | 0 |
| Spleen volume at Week 24 not available | 4 ( 57.1%) | 1 ( 16.7%) | 5 ( 38.5%) |
| >0% spleen volume increase at Week 24 | 1 ( 14.3%) | 2 ( 33.3%) | 3 ( 23.1%) |
| <35% spleen volume reduction at Week 24 | 3 ( 42.9%) | 4 ( 66.7%) | 7 ( 53.8%) |
| Last participation date < Day 141 in RT phase | 3 ( 42.9%) | 1 ( 16.7%) | 4 ( 30.8%) |
| **Splenic Response Rate at Any Timepoint in RT Phase** | | | |
| Responder, n(%) | 0 | 1 ( 16.7%) | 1 ( 7.7%) |
| 95% Exact CI | 0.0000, 0.4096 | 0.0042, 0.6412 | 0.0019, 0.3603 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Baseline TSS >=18 | 7 | 8 | 15 |
| Splenic Response Rate at Week 12 | | | |
| Responder, n(%) | 0 | 0 | 0 |
| 95% Exact CI | 0.0000, 0.4096 | 0.0000, 0.3694 | 0.0000, 0.2180 |
| Proportion Difference (95% CI) | 0.00 (-0.26, 0.26) | | |
| Proportion Difference using Exact Method (95% CI) | | | |
| Non-Responder, n(%) | 7 (100.0%) | 8 (100.0%) | 15 (100.0%) |
| Baseline spleen volume not available | 0 | 0 | 0 |
| Spleen volume at Week 12 not available | 2 ( 28.6%) | 3 ( 37.5%) | 5 ( 33.3%) |
| >0% spleen volume increase at Week 12 | 1 ( 14.3%) | 3 ( 37.5%) | 4 ( 26.7%) |
| <35% spleen volume reduction at Week 12 | 5 ( 71.4%) | 5 ( 62.5%) | 10 ( 66.7%) |
| Last participation date < Day 57 in RT phase | 1 ( 14.3%) | 0 | 1 ( 6.7%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

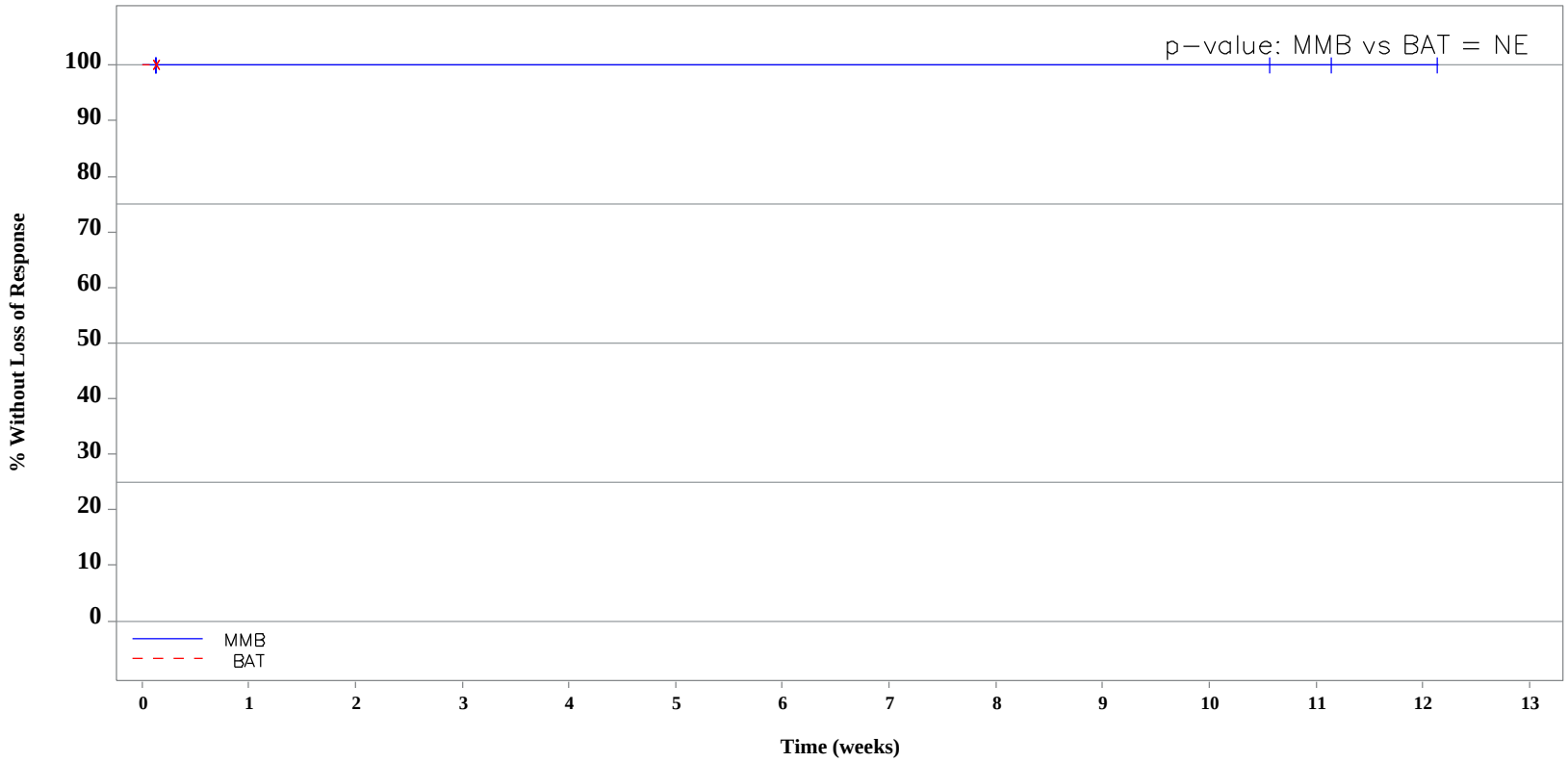
Table 2.0101: Analysis of Splenic Response Rate at Week 12 and at Week 24
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **Splenic Response Rate at Week 24** | | | |
| Responder, n(%) | 1 ( 14.3%) | 0 | 1 ( 6.7%) |
| 95% Exact CI | 0.0036, 0.5787 | 0.0000, 0.3694 | 0.0017, 0.3195 |
| Proportion Difference (95% CI) | 0.14 (-0.21, 0.50) | | |
| Proportion Difference using Exact Method (95% CI) | 0.14 (-0.36, 0.58) | | |
| Non-Responder, n(%) | 6 ( 85.7%) | 8 (100.0%) | 14 ( 93.3%) |
| Baseline spleen volume not available | 0 | 0 | 0 |
| Spleen volume at Week 24 not available | 3 ( 42.9%) | 2 ( 25.0%) | 5 ( 33.3%) |
| >0% spleen volume increase at Week 24 | 2 ( 28.6%) | 3 ( 37.5%) | 5 ( 33.3%) |
| <35% spleen volume reduction at Week 24 | 3 ( 42.9%) | 6 ( 75.0%) | 9 ( 60.0%) |
| Last participation date < Day 141 in RT phase | 3 ( 42.9%) | 2 ( 25.0%) | 5 ( 33.3%) |
| **Splenic Response Rate at Any Timepoint in RT Phase** | | | |
| Responder, n(%) | 1 ( 14.3%) | 0 | 1 ( 6.7%) |
| 95% Exact CI | 0.0036, 0.5787 | 0.0000, 0.3694 | 0.0017, 0.3195 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
Modified Poisson regression model did not converge so these values are presented as NE. NE = Not Estimable.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Figure 2.0302: Kaplan-Meier Plot of Duration of MRI/CT Splenic Response
Randomized Treatment Phase
ITT-Anemic Analysis Set with >= 35% Spleen Volume Reduction from Baseline in RT phase




**N at Risk (Events)**

| | | | | | | | | | | | | | | |
|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|
| **MMB** | 6 (0) | 3 (0) | 3 (0) | 3 (0) | 3 (0) | 3 (0) | 3 (0) | 3 (0) | 3 (0) | 3 (0) | 3 (0) | 2 (0) | 1 (0) | 0 (0) |
| **BAT** | 2 (0) | 0 (0) | 0 (0) | 0 (0) | 0 (0) | 0 (0) | 0 (0) | 0 (0) | 0 (0) | 0 (0) | 0 (0) | 0 (0) | 0 (0) | 0 (0) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL. NE = Not estimable.
Stratified two-sided p-value is from a log-rank test comparing treatment stratified by baseline TD (yes,no) and baseline TSS (<18,>=18).

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/g-dur-sr-rt.sas V.03.05 Output file: g-dur-sr35-rt.pdf 17OCT2023:20:35

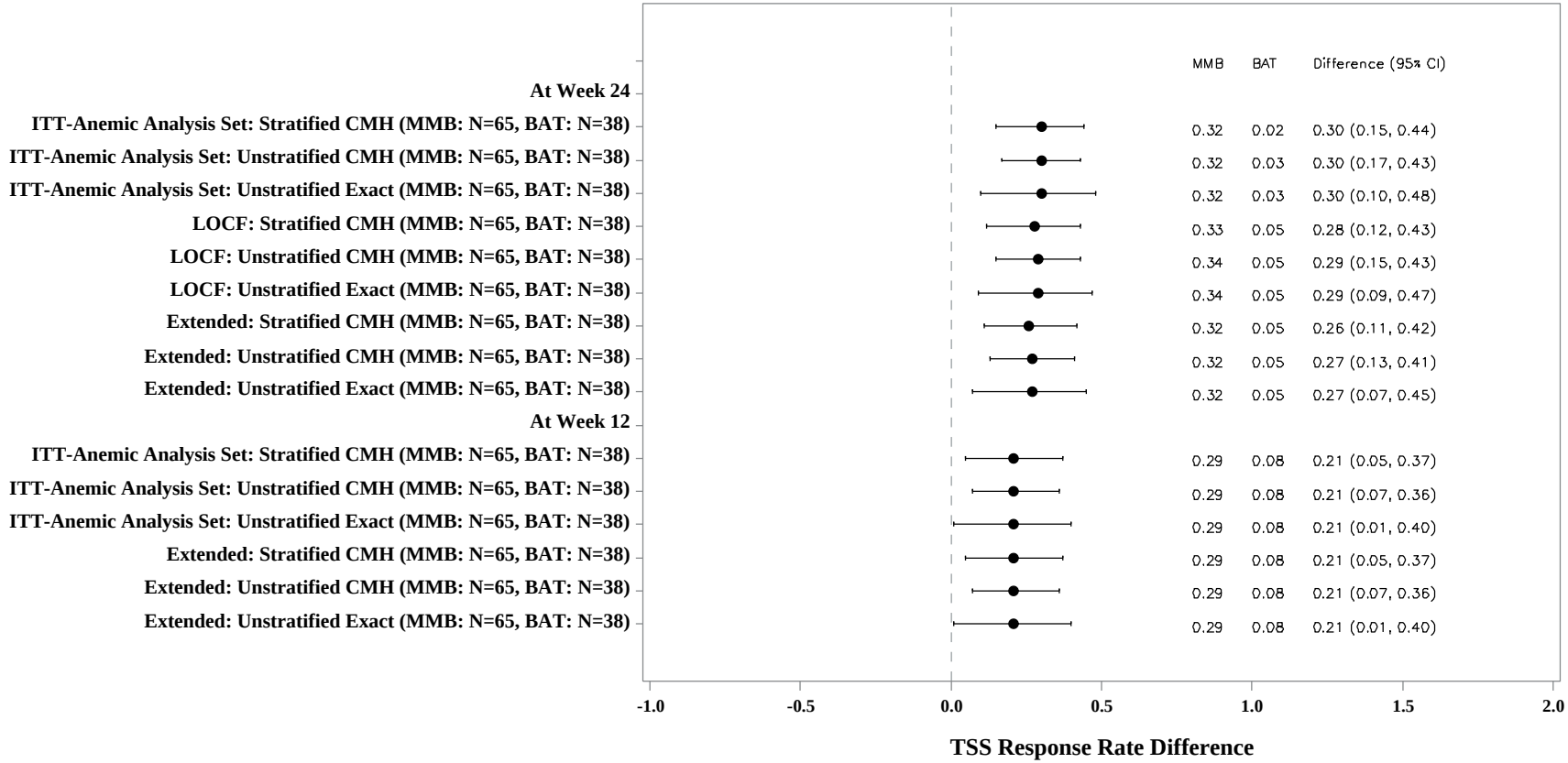
Table 2.0811: Analysis of Duration of MRI/CT Splenic Response
Randomized Treatment Phase
ITT-Anemic Analysis Set with >= 35% Spleen Volume Reduction from Baseline in RT phase

| | MMB (N=6) | BAT (N=2) |
|---|---|---|
| **Subjects with Event** | | |
| Loss of spleen response, n(%) | 0 | 0 |
| **Censor** | | |
| Subjects Censored, n(%) | 6 (100.0%) | 2 (100.0%) |
| Non-Responder: Censored at Randomization Date | 0 | 0 |
| No Loss of Response: Censored at Last MRI Spleen Assessment Date in RT phase (including up to 10 days after First Dose Date of ET phase) | 6 (100.0%) | 2 (100.0%) |
| **Kaplan-Meier Estimate of Duration of Spleen Response (Weeks)** | | |
| 25-percentile (95% CI) | NA | NA |
| Median (95% CI) | NA | NA |
| 75-percentile (95% CI) | NA | NA |
| Min, Max | 0.14, 12.14 | 0.14, 0.14 |
| **Stratified Log-Rank Test p-value** | NA | |
| **Adjusted Hazard Ratio (95% CI)** | NA | |
| **Unstratified Log-Rank Test p-value** | NA | |
| **Unadjusted Hazard Ratio (95% CI)** | NA | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
Stratified two-sided p-value is from a log-rank test comparing treatment stratified by baseline TD (yes,no) and baseline TSS (<18,>=18).
Adjusted hazard ratio and 95% CI are from a Cox proportional hazards model with covariates of treatment, baseline TD (yes,no) and baseline TSS (<18,>=18). Unstratified two-sided p-value is from a log-rank test comparing treatment.
Unadjusted hazard ratio and 95% CI are from a Cox proportional hazards model with a covariate of treatment.
MRI = Magnetic Resonance Imaging. CT = Computerized Tomography.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-dur-sr35.sas V.03.05 Output file: t-dur-sr35.pdf 17OCT2023:18:03

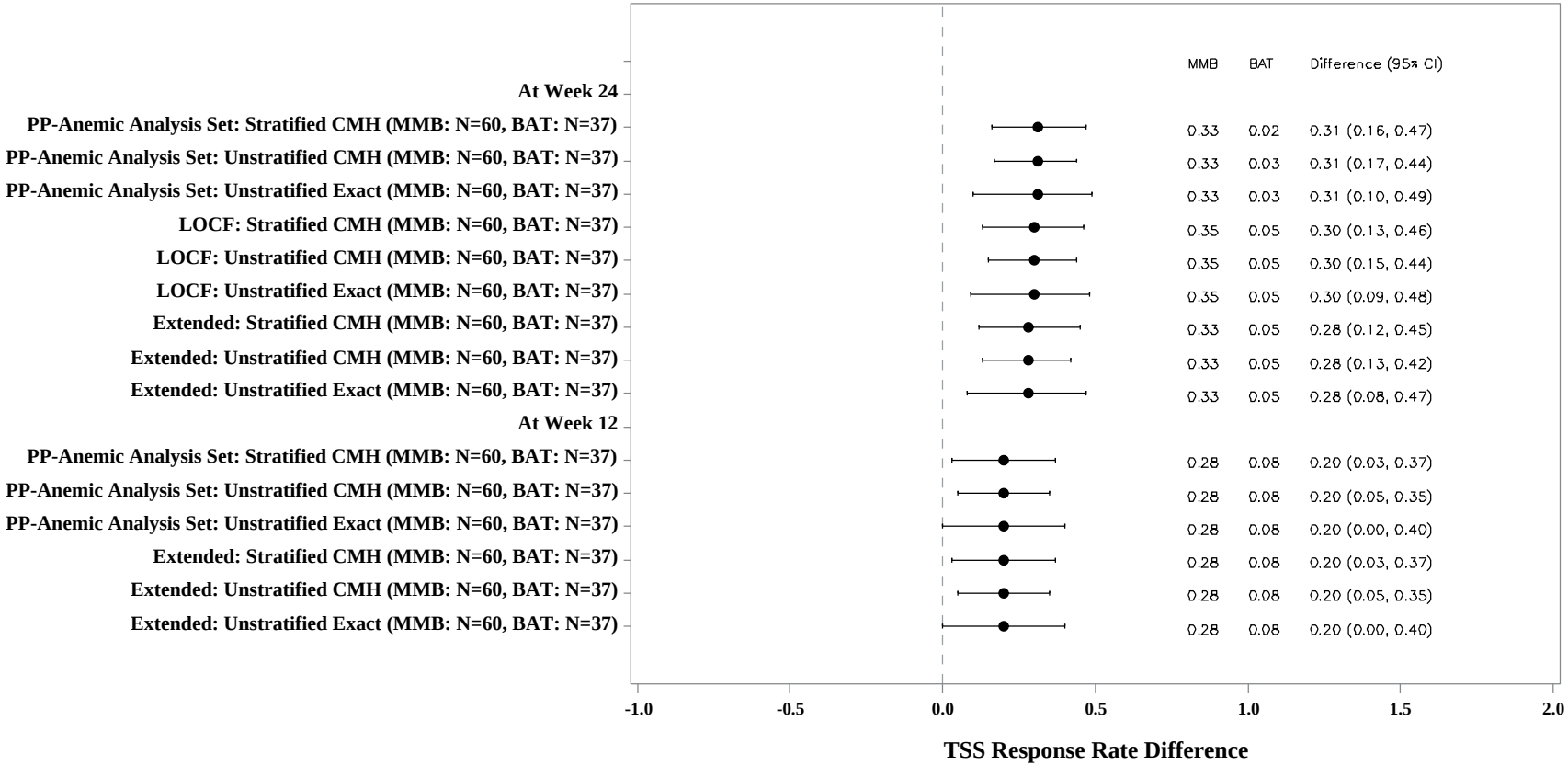
Figure 2.1301: Forest plot of Primary and Sensitivity Analysis of Response Rate in TSS at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set
All strata combined




ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
To the right of the reference line favors MMB, to the left favors BAT.
CMH = Cochran-Mantel-Haenszel; LOCF = Last Observation Carried Forward; CI= Confidence Interval
TSS = Total Symptom Score.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/g-tss-forest.sas V.03.05 Output file: g-tss-forest.pdf 25AUG2023:13:35

Figure 2.1302: Forest plot of Primary and Sensitivity Analysis of Response Rate in TSS at Week 24 and Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set
All strata combined




PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
To the right of the reference line favors MMB, to the left favors BAT.
CMH = Cochran-Mantel-Haenszel; LOCF = Last Observation Carried Forward; PP=Per-Protocol; CI= Confidence Interval.
TSS = Total Symptom Score.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/g-tss-forest.sas V.03.05 Output file: g-tss-forest-pp.pdf 25AUG2023:13:36

Table 2.2301: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| All Strata Combined | | | |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 1 ( 1.5%) | 3 ( 7.7%) | 4 ( 3.8%) |
| TSS > 0 at baseline | 65 ( 98.5%) | 36 ( 92.3%) | 101 ( 96.2%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 65 | 38 | 103 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 2 ( 5.3%) | 2 ( 1.9%) |
| Responder, n(%) | 21 ( 32.3%) | 2 ( 5.3%) | 23 ( 22.3%) |
| 95% Exact CI | 0.2123, 0.4505 | 0.0064, 0.1775 | 0.1471, 0.3160 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.26 (0.11, 0.42) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.27 (0.13, 0.41) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.27 (0.07, 0.45) | | |
| p-value | 0.001 | | |
| Non-Responder, n(%) | 44 ( 67.7%) | 36 ( 94.7%) | 80 ( 77.7%) |
| Last participation date < Day 162 in RT Phase | 18 ( 27.7%) | 7 ( 18.4%) | 25 ( 24.3%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 1 ( 1.5%) | 1 ( 2.6%) | 2 ( 1.9%) |
| >0% increase from baseline at Week 24 | 15 ( 23.1%) | 17 ( 44.7%) | 32 ( 31.1%) |
| <50% reduction from baseline at Week 24 | 25 ( 38.5%) | 27 ( 71.1%) | 52 ( 50.5%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Extended Criteria sensitivity analysis: Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92). Day 1 is defined as the date of randomization.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 25JUN2019

Table 2.2301: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 65 | 38 | 103 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 2 ( 5.3%) | 2 ( 1.9%) |
| Responder, n(%) | 19 ( 29.2%) | 3 ( 7.9%) | 22 ( 21.4%) |
| 95% Exact CI | 0.1860, 0.4183 | 0.0166, 0.2138 | 0.1390, 0.3053 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.21 (0.05, 0.37) | | |
| p-value | 0.010 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.21 (0.07, 0.36) | | |
| p-value | 0.004 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.21 (0.01, 0.40) | | |
| p-value | 0.012 | | |
| Non-Responder, n(%) | 46 ( 70.8%) | 35 ( 92.1%) | 81 ( 78.6%) |
| Last participation date < Day 78 in RT Phase | 7 ( 10.8%) | 6 ( 15.8%) | 13 ( 12.6%) |
| Last participation date >= Day 78 and TSS at Week 12 not available | 1 ( 1.5%) | 0 | 1 ( 1.0%) |
| >0% increase from baseline at Week 12 | 19 ( 29.2%) | 16 ( 42.1%) | 35 ( 34.0%) |
| <50% reduction from baseline at Week 12 | 38 ( 58.5%) | 27 ( 71.1%) | 65 ( 63.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Extended Criteria sensitivity analysis: Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92). Day 1 is defined as the date of randomization.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 25JUN2019

Table 2.2301: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Strata 1 | | | |
| Transfusion Dependence Yes and Baseline TSS <18 | 35 | 14 | 49 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 0 | 2 ( 14.3%) | 2 ( 4.1%) |
| TSS > 0 at baseline | 35 (100.0%) | 12 ( 85.7%) | 47 ( 95.9%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 35 | 14 | 49 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 2 ( 14.3%) | 2 ( 4.1%) |
| Responder, n(%) | 12 ( 34.3%) | 1 ( 7.1%) | 13 ( 26.5%) |
| 95% Exact CI | 0.1913, 0.5221 | 0.0018, 0.3387 | 0.1495, 0.4108 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.27 (0.04, 0.50) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.27 (-0.05, 0.56) | | |
| Non-Responder, n(%) | 23 ( 65.7%) | 13 ( 92.9%) | 36 ( 73.5%) |
| Last participation date < Day 162 in RT Phase | 6 ( 17.1%) | 2 ( 14.3%) | 8 ( 16.3%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 1 ( 2.9%) | 1 ( 7.1%) | 2 ( 4.1%) |
| >0% increase from baseline at Week 24 | 11 ( 31.4%) | 7 ( 50.0%) | 18 ( 36.7%) |
| <50% reduction from baseline at Week 24 | 16 ( 45.7%) | 9 ( 64.3%) | 25 ( 51.0%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Extended Criteria sensitivity analysis: Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92). Day 1 is defined as the date of randomization.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-tss24.sas V.03.05 Output file: t-sen-ext-tss24.pdf 24AUG2023:16:39

Table 2.2301: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 35 | 13 | 48 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 1 ( 7.7%) | 1 ( 2.1%) |
| Responder, n(%) | 11 ( 31.4%) | 1 ( 7.7%) | 12 ( 25.0%) |
| 95% Exact CI | 0.1685, 0.4929 | 0.0019, 0.3603 | 0.1364, 0.3960 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.24 (0.00, 0.47) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.24 (-0.08, 0.54) | | |
| Non-Responder, n(%) | 24 ( 68.6%) | 12 ( 92.3%) | 36 ( 75.0%) |
| Last participation date < Day 78 in RT Phase | 1 ( 2.9%) | 2 ( 15.4%) | 3 ( 6.3%) |
| Last participation date >= Day 78 and TSS at Week 12 not available | 1 ( 2.9%) | 0 | 1 ( 2.1%) |
| >0% increase from baseline at Week 12 | 11 ( 31.4%) | 6 ( 46.2%) | 17 ( 35.4%) |
| <50% reduction from baseline at Week 12 | 22 ( 62.9%) | 9 ( 69.2%) | 31 ( 64.6%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Extended Criteria sensitivity analysis: Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92). Day 1 is defined as the date of randomization.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 25JUN2019

Table 2.2301: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Strata 2 | | | |
| Transfusion Dependence Yes and Baseline TSS >=18 | 17 | 11 | 28 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 17 (100.0%) | 11 (100.0%) | 28 (100.0%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 17 | 11 | 28 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 6 ( 35.3%) | 0 | 6 ( 21.4%) |
| 95% Exact CI | 0.1421, 0.6167 | 0.0000, 0.2849 | 0.0830, 0.4095 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.35 (0.09, 0.62) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.35 (-0.02, 0.66) | | |
| Non-Responder, n(%) | 11 ( 64.7%) | 11 (100.0%) | 22 ( 78.6%) |
| Last participation date < Day 162 in RT Phase | 6 ( 35.3%) | 2 ( 18.2%) | 8 ( 28.6%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 24 | 2 ( 11.8%) | 7 ( 63.6%) | 9 ( 32.1%) |
| <50% reduction from baseline at Week 24 | 5 ( 29.4%) | 9 ( 81.8%) | 14 ( 50.0%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Extended Criteria sensitivity analysis: Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92). Day 1 is defined as the date of randomization.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 25JUN2019

Table 2.2301: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 17 | 11 | 28 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 0 | 0 |
| Responder, n(%) | 4 ( 23.5%) | 1 ( 9.1%) | 5 ( 17.9%) |
| 95% Exact CI | 0.0681, 0.4990 | 0.0023, 0.4128 | 0.0606, 0.3689 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.14 (-0.15, 0.44) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.14 (-0.23, 0.49) | | |
| Non-Responder, n(%) | 13 ( 76.5%) | 10 ( 90.9%) | 23 ( 82.1%) |
| Last participation date < Day 78 in RT Phase | 3 ( 17.6%) | 2 ( 18.2%) | 5 ( 17.9%) |
| Last participation date >= Day 78 and TSS at Week 12 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 12 | 4 ( 23.5%) | 6 ( 54.5%) | 10 ( 35.7%) |
| <50% reduction from baseline at Week 12 | 10 ( 58.8%) | 8 ( 72.7%) | 18 ( 64.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Extended Criteria sensitivity analysis: Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92). Day 1 is defined as the date of randomization.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 25JUN2019

Table 2.2301: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Strata 3 | | | |
| Transfusion Dependence No and Baseline TSS <18 | 7 | 6 | 13 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 1 ( 14.3%) | 1 ( 16.7%) | 2 ( 15.4%) |
| TSS > 0 at baseline | 6 ( 85.7%) | 5 ( 83.3%) | 11 ( 84.6%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 6 | 5 | 11 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 1 ( 16.7%) | 0 | 1 ( 9.1%) |
| 95% Exact CI | 0.0042, 0.6412 | 0.0000, 0.5218 | 0.0023, 0.4128 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.17 (-0.28, 0.62) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.17 (-0.39, 0.67) | | |
| Non-Responder, n(%) | 5 ( 83.3%) | 5 (100.0%) | 10 ( 90.9%) |
| Last participation date < Day 162 in RT Phase | 3 ( 50.0%) | 1 ( 20.0%) | 4 ( 36.4%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 24 | 1 ( 16.7%) | 1 ( 20.0%) | 2 ( 18.2%) |
| <50% reduction from baseline at Week 24 | 2 ( 33.3%) | 4 ( 80.0%) | 6 ( 54.5%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Extended Criteria sensitivity analysis: Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92). Day 1 is defined as the date of randomization.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 25JUN2019

Table 2.2301: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 6 | 6 | 12 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 1 ( 16.7%) | 1 ( 8.3%) |
| Responder, n(%) | 1 ( 16.7%) | 0 | 1 ( 8.3%) |
| 95% Exact CI | 0.0042, 0.6412 | 0.0000, 0.4593 | 0.0021, 0.3848 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.17 (-0.26, 0.59) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.17 (-0.45, 0.70) | | |
| Non-Responder, n(%) | 5 ( 83.3%) | 6 (100.0%) | 11 ( 91.7%) |
| Last participation date < Day 78 in RT Phase | 2 ( 33.3%) | 0 | 2 ( 16.7%) |
| Last participation date >= Day 78 and TSS at Week 12 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 12 | 3 ( 50.0%) | 2 ( 33.3%) | 5 ( 41.7%) |
| <50% reduction from baseline at Week 12 | 3 ( 50.0%) | 5 ( 83.3%) | 8 ( 66.7%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Extended Criteria sensitivity analysis: Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92). Day 1 is defined as the date of randomization.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 25JUN2019

Table 2.2301: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Strata 4 | | | |
| Transfusion Dependence No and Baseline TSS >=18 | 7 | 8 | 15 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 7 (100.0%) | 8 (100.0%) | 15 (100.0%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 7 | 8 | 15 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 2 ( 28.6%) | 1 ( 12.5%) | 3 ( 20.0%) |
| 95% Exact CI | 0.0367, 0.7096 | 0.0032, 0.5265 | 0.0433, 0.4809 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.16 (-0.30, 0.62) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.16 (-0.36, 0.60) | | |
| Non-Responder, n(%) | 5 ( 71.4%) | 7 ( 87.5%) | 12 ( 80.0%) |
| Last participation date < Day 162 in RT Phase | 3 ( 42.9%) | 2 ( 25.0%) | 5 ( 33.3%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 24 | 1 ( 14.3%) | 2 ( 25.0%) | 3 ( 20.0%) |
| <50% reduction from baseline at Week 24 | 2 ( 28.6%) | 5 ( 62.5%) | 7 ( 46.7%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Extended Criteria sensitivity analysis: Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92). Day 1 is defined as the date of randomization.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 25JUN2019

Table 2.2301: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12 -- Extended Criteria
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 7 | 8 | 15 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 0 | 0 |
| Responder, n(%) | 3 ( 42.9%) | 1 ( 12.5%) | 4 ( 26.7%) |
| 95% Exact CI | 0.0990, 0.8159 | 0.0032, 0.5265 | 0.0779, 0.5510 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.30 (-0.18, 0.79) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.30 (-0.23, 0.72) | | |
| Non-Responder, n(%) | 4 ( 57.1%) | 7 ( 87.5%) | 11 ( 73.3%) |
| Last participation date < Day 78 in RT Phase | 1 ( 14.3%) | 2 ( 25.0%) | 3 ( 20.0%) |
| Last participation date >= Day 78 and TSS at Week 12 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 12 | 1 ( 14.3%) | 2 ( 25.0%) | 3 ( 20.0%) |
| <50% reduction from baseline at Week 12 | 3 ( 42.9%) | 5 ( 62.5%) | 8 ( 53.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Extended Criteria sensitivity analysis: Baseline TSS is defined as the average of >= 1 daily TSS from the 7-day period at Baseline.
TSS at Week 24 (12) is the last available consecutive 28 days with >= 4 daily TSS prior to Day 176(92). Day 1 is defined as the date of randomization.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 25JUN2019

Table 2.2201: Sensitivity Analysis of Response Rate in TSS at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| All Strata Combined | | | |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 1 ( 1.5%) | 3 ( 7.7%) | 4 ( 3.8%) |
| TSS > 0 at baseline | 65 ( 98.5%) | 36 ( 92.3%) | 101 ( 96.2%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 65 | 38 | 103 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 2 ( 5.3%) | 2 ( 1.9%) |
| Responder, n(%) | 22 ( 33.8%) | 2 ( 5.3%) | 24 ( 23.3%) |
| 95% Exact CI | 0.2257, 0.4665 | 0.0064, 0.1775 | 0.1554, 0.3266 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.28 (0.12, 0.43) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.29 (0.15, 0.43) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.29 (0.09, 0.47) | | |
| p-value | <0.001 | | |
| Non-Responder, n(%) | 43 ( 66.2%) | 36 ( 94.7%) | 79 ( 76.7%) |
| Last participation date < Day 162 in RT Phase | 18 ( 27.7%) | 7 ( 18.4%) | 25 ( 24.3%) |
| TSS at Week 24 not available | 5 ( 7.7%) | 6 ( 15.8%) | 11 ( 10.7%) |
| >0% increase from baseline at Week 24 | 23 ( 35.4%) | 18 ( 47.4%) | 41 ( 39.8%) |
| <50% reduction from baseline at Week 24 | 38 ( 58.5%) | 28 ( 73.7%) | 66 ( 64.1%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
LOCF = Last Observation Carried Forward
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 25JUN2019

Table 2.2201: Sensitivity Analysis of Response Rate in TSS at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Strata 1 | | | |
| Transfusion Dependence Yes and Baseline TSS <18 | 35 | 14 | 49 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 0 | 2 ( 14.3%) | 2 ( 4.1%) |
| TSS > 0 at baseline | 35 (100.0%) | 12 ( 85.7%) | 47 ( 95.9%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 35 | 14 | 49 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 2 ( 14.3%) | 2 ( 4.1%) |
| Responder, n(%) | 13 ( 37.1%) | 1 ( 7.1%) | 14 ( 28.6%) |
| 95% Exact CI | 0.2147, 0.5508 | 0.0018, 0.3387 | 0.1658, 0.4326 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.30 (0.07, 0.53) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.30 (-0.02, 0.58) | | |
| Non-Responder, n(%) | 22 ( 62.9%) | 13 ( 92.9%) | 35 ( 71.4%) |
| Last participation date < Day 162 in RT Phase | 6 ( 17.1%) | 2 ( 14.3%) | 8 ( 16.3%) |
| TSS at Week 24 not available | 2 ( 5.7%) | 2 ( 14.3%) | 4 ( 8.2%) |
| >0% increase from baseline at Week 24 | 13 ( 37.1%) | 7 ( 50.0%) | 20 ( 40.8%) |
| <50% reduction from baseline at Week 24 | 20 ( 57.1%) | 9 ( 64.3%) | 29 ( 59.2%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
LOCF = Last Observation Carried Forward
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 25JUN2019

Table 2.2201: Sensitivity Analysis of Response Rate in TSS at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Strata 2 | | | |
| Transfusion Dependence Yes and Baseline TSS >=18 | 17 | 11 | 28 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 17 (100.0%) | 11 (100.0%) | 28 (100.0%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 17 | 11 | 28 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 6 ( 35.3%) | 0 | 6 ( 21.4%) |
| 95% Exact CI | 0.1421, 0.6167 | 0.0000, 0.2849 | 0.0830, 0.4095 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.35 (0.09, 0.62) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.35 (-0.02, 0.66) | | |
| Non-Responder, n(%) | 11 ( 64.7%) | 11 (100.0%) | 22 ( 78.6%) |
| Last participation date < Day 162 in RT Phase | 6 ( 35.3%) | 2 ( 18.2%) | 8 ( 28.6%) |
| TSS at Week 24 not available | 2 ( 11.8%) | 2 ( 18.2%) | 4 ( 14.3%) |
| >0% increase from baseline at Week 24 | 6 ( 35.3%) | 7 ( 63.6%) | 13 ( 46.4%) |
| <50% reduction from baseline at Week 24 | 9 ( 52.9%) | 9 ( 81.8%) | 18 ( 64.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
LOCF = Last Observation Carried Forward
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 25JUN2019

Table 2.2201: Sensitivity Analysis of Response Rate in TSS at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Strata 3 | | | |
| Transfusion Dependence No and Baseline TSS <18 | 7 | 6 | 13 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 1 ( 14.3%) | 1 ( 16.7%) | 2 ( 15.4%) |
| TSS > 0 at baseline | 6 ( 85.7%) | 5 ( 83.3%) | 11 ( 84.6%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 6 | 5 | 11 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 1 ( 16.7%) | 0 | 1 ( 9.1%) |
| 95% Exact CI | 0.0042, 0.6412 | 0.0000, 0.5218 | 0.0023, 0.4128 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.17 (-0.28, 0.62) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.17 (-0.39, 0.67) | | |
| Non-Responder, n(%) | 5 ( 83.3%) | 5 (100.0%) | 10 ( 90.9%) |
| Last participation date < Day 162 in RT Phase | 3 ( 50.0%) | 1 ( 20.0%) | 4 ( 36.4%) |
| TSS at Week 24 not available | 1 ( 16.7%) | 1 ( 20.0%) | 2 ( 18.2%) |
| >0% increase from baseline at Week 24 | 2 ( 33.3%) | 1 ( 20.0%) | 3 ( 27.3%) |
| <50% reduction from baseline at Week 24 | 4 ( 66.7%) | 4 ( 80.0%) | 8 ( 72.7%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
LOCF = Last Observation Carried Forward
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 25JUN2019

Table 2.2201: Sensitivity Analysis of Response Rate in TSS at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Strata 4 | | | |
| Transfusion Dependence No and Baseline TSS >=18 | 7 | 8 | 15 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 7 (100.0%) | 8 (100.0%) | 15 (100.0%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 7 | 8 | 15 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 2 ( 28.6%) | 1 ( 12.5%) | 3 ( 20.0%) |
| 95% Exact CI | 0.0367, 0.7096 | 0.0032, 0.5265 | 0.0433, 0.4809 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.16 (-0.30, 0.62) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.16 (-0.36, 0.60) | | |
| Non-Responder, n(%) | 5 ( 71.4%) | 7 ( 87.5%) | 12 ( 80.0%) |
| Last participation date < Day 162 in RT Phase | 3 ( 42.9%) | 2 ( 25.0%) | 5 ( 33.3%) |
| TSS at Week 24 not available | 0 | 1 ( 12.5%) | 1 (  6.7%) |
| >0% increase from baseline at Week 24 | 2 ( 28.6%) | 3 ( 37.5%) | 5 ( 33.3%) |
| <50% reduction from baseline at Week 24 | 5 ( 71.4%) | 6 ( 75.0%) | 11 ( 73.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
LOCF = Last Observation Carried Forward
TSS = Total Symptom Score;  CMH = Cochran-Mantel-Haenszel;

Data Extracted: CRF data: 25JUN2019

Table 2.2202: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| All Strata Combined | | | |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 1 ( 1.6%) | 3 ( 7.9%) | 4 ( 4.0%) |
| TSS > 0 at baseline | 60 ( 98.4%) | 35 ( 92.1%) | 95 ( 96.0%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 60 | 37 | 97 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 2 ( 5.4%) | 2 ( 2.1%) |
| Responder, n(%) | 20 ( 33.3%) | 1 ( 2.7%) | 21 ( 21.6%) |
| 95% Exact CI | 0.2169, 0.4669 | 0.0007, 0.1416 | 0.1393, 0.3117 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.31 (0.16, 0.47) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.31 (0.17, 0.44) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.31 (0.10, 0.49) | | |
| p-value | <0.001 | | |
| Non-Responder, n(%) | 40 ( 66.7%) | 36 ( 97.3%) | 76 ( 78.4%) |
| Last participation date < Day 162 in RT Phase | 16 ( 26.7%) | 7 ( 18.9%) | 23 ( 23.7%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 1 ( 1.7%) | 3 ( 8.1%) | 4 ( 4.1%) |
| >0% increase from baseline at Week 24 | 13 ( 21.7%) | 16 ( 43.2%) | 29 ( 29.9%) |
| <50% reduction from baseline at Week 24 | 23 ( 38.3%) | 25 ( 67.6%) | 48 ( 49.5%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel; PP = Per-Protocol.

Data Extracted: CRF data: 25JUN2019

Table 2.2202: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 60 | 37 | 97 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 2 ( 5.4%) | 2 ( 2.1%) |
| Responder, n(%) | 17 ( 28.3%) | 3 ( 8.1%) | 20 ( 20.6%) |
| 95% Exact CI | 0.1745, 0.4144 | 0.0170, 0.2191 | 0.1307, 0.3003 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.20 (0.03, 0.37) | | |
| p-value | 0.018 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.20 (0.05, 0.35) | | |
| p-value | 0.008 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.20 (0.00, 0.40) | | |
| p-value | 0.020 | | |
| Non-Responder, n(%) | 43 ( 71.7%) | 34 ( 91.9%) | 77 ( 79.4%) |
| Last participation date < Day 78 in RT Phase | 6 ( 10.0%) | 6 ( 16.2%) | 12 ( 12.4%) |
| Last participation date >= Day 78 and TSS at Week 12 not available | 1 ( 1.7%) | 0 | 1 ( 1.0%) |
| >0% increase from baseline at Week 12 | 18 ( 30.0%) | 15 ( 40.5%) | 33 ( 34.0%) |
| <50% reduction from baseline at Week 12 | 36 ( 60.0%) | 26 ( 70.3%) | 62 ( 63.9%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel; PP = Per-Protocol.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-tss24.sas V.03.05 Output file: t-sen-ppt-tss24.pdf 24AUG2023:16:38

Table 2.2202: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Strata 1 | | | |
| Transfusion Dependence Yes and Baseline TSS <18 | 32 | 14 | 46 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 0 | 2 ( 14.3%) | 2 ( 4.3%) |
| TSS > 0 at baseline | 32 (100.0%) | 12 ( 85.7%) | 44 ( 95.7%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 32 | 14 | 46 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 2 ( 14.3%) | 2 ( 4.3%) |
| Responder, n(%) | 11 ( 34.4%) | 0 | 11 ( 23.9%) |
| 95% Exact CI | 0.1857, 0.5319 | 0.0000, 0.2316 | 0.1259, 0.3877 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.34 (0.15, 0.54) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.34 (0.03, 0.62) | | |
| Non-Responder, n(%) | 21 ( 65.6%) | 14 (100.0%) | 35 ( 76.1%) |
| Last participation date < Day 162 in RT Phase | 6 ( 18.8%) | 2 ( 14.3%) | 8 ( 17.4%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 1 ( 3.1%) | 2 ( 14.3%) | 3 ( 6.5%) |
| >0% increase from baseline at Week 24 | 9 ( 28.1%) | 7 ( 50.0%) | 16 ( 34.8%) |
| <50% reduction from baseline at Week 24 | 14 ( 43.8%) | 9 ( 64.3%) | 23 ( 50.0%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel; PP = Per-Protocol.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-tss24.sas V.03.05 Output file: t-sen-ppt-tss24.pdf 24AUG2023:16:38

Table 2.2202: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 32 | 13 | 45 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 1 ( 7.7%) | 1 ( 2.2%) |
| Responder, n(%) | 10 ( 31.3%) | 1 ( 7.7%) | 11 ( 24.4%) |
| 95% Exact CI | 0.1612, 0.5001 | 0.0019, 0.3603 | 0.1288, 0.3954 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.24 (0.00, 0.48) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.24 (-0.09, 0.54) | | |
| Non-Responder, n(%) | 22 ( 68.8%) | 12 ( 92.3%) | 34 ( 75.6%) |
| Last participation date < Day 78 in RT Phase | 1 ( 3.1%) | 2 ( 15.4%) | 3 ( 6.7%) |
| Last participation date >= Day 78 and TSS at Week 12 not available | 1 ( 3.1%) | 0 | 1 ( 2.2%) |
| >0% increase from baseline at Week 12 | 10 ( 31.3%) | 6 ( 46.2%) | 16 ( 35.6%) |
| <50% reduction from baseline at Week 12 | 20 ( 62.5%) | 9 ( 69.2%) | 29 ( 64.4%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel; PP = Per-Protocol.

Data Extracted: CRF data: 25JUN2019

Table 2.2202: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Strata 2 | | | |
| Transfusion Dependence Yes and Baseline TSS >=18 | 17 | 10 | 27 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 17 (100.0%) | 10 (100.0%) | 27 (100.0%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 17 | 10 | 27 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 6 ( 35.3%) | 0 | 6 ( 22.2%) |
| 95% Exact CI | 0.1421, 0.6167 | 0.0000, 0.3085 | 0.0862, 0.4226 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.35 (0.08, 0.62) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.35 (-0.03, 0.68) | | |
| Non-Responder, n(%) | 11 ( 64.7%) | 10 (100.0%) | 21 ( 77.8%) |
| Last participation date < Day 162 in RT Phase | 6 ( 35.3%) | 2 ( 20.0%) | 8 ( 29.6%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 0 | 1 ( 10.0%) | 1 ( 3.7%) |
| >0% increase from baseline at Week 24 | 2 ( 11.8%) | 6 ( 60.0%) | 8 ( 29.6%) |
| <50% reduction from baseline at Week 24 | 5 ( 29.4%) | 7 ( 70.0%) | 12 ( 44.4%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel; PP = Per-Protocol.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-tss24.sas V.03.05 Output file: t-sen-ppt-tss24.pdf 24AUG2023:16:38

Table 2.2202: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 17 | 10 | 27 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 0 | 0 |
| Responder, n(%) | 4 ( 23.5%) | 1 ( 10.0%) | 5 ( 18.5%) |
| 95% Exact CI | 0.0681, 0.4990 | 0.0025, 0.4450 | 0.0630, 0.3808 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.14 (-0.17, 0.44) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.14 (-0.25, 0.49) | | |
| Non-Responder, n(%) | 13 ( 76.5%) | 9 ( 90.0%) | 22 ( 81.5%) |
| Last participation date < Day 78 in RT Phase | 3 ( 17.6%) | 2 ( 20.0%) | 5 ( 18.5%) |
| Last participation date >= Day 78 and TSS at Week 12 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 12 | 4 ( 23.5%) | 5 ( 50.0%) | 9 ( 33.3%) |
| <50% reduction from baseline at Week 12 | 10 ( 58.8%) | 7 ( 70.0%) | 17 ( 63.0%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel; PP = Per-Protocol.

Data Extracted: CRF data: 25JUN2019

Table 2.2202: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Strata 3 | | | |
| Transfusion Dependence No and Baseline TSS <18 | 7 | 6 | 13 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 1 ( 14.3%) | 1 ( 16.7%) | 2 ( 15.4%) |
| TSS > 0 at baseline | 6 ( 85.7%) | 5 ( 83.3%) | 11 ( 84.6%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 6 | 5 | 11 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 1 ( 16.7%) | 0 | 1 ( 9.1%) |
| 95% Exact CI | 0.0042, 0.6412 | 0.0000, 0.5218 | 0.0023, 0.4128 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.17 (-0.28, 0.62) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.17 (-0.39, 0.67) | | |
| Non-Responder, n(%) | 5 ( 83.3%) | 5 (100.0%) | 10 ( 90.9%) |
| Last participation date < Day 162 in RT Phase | 3 ( 50.0%) | 1 ( 20.0%) | 4 ( 36.4%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 24 | 1 ( 16.7%) | 1 ( 20.0%) | 2 ( 18.2%) |
| <50% reduction from baseline at Week 24 | 2 ( 33.3%) | 4 ( 80.0%) | 6 ( 54.5%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel; PP = Per-Protocol.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-tss24.sas V.03.05 Output file: t-sen-ppt-tss24.pdf 24AUG2023:16:38

Table 2.2202: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 6 | 6 | 12 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 1 ( 16.7%) | 1 ( 8.3%) |
| Responder, n(%) | 1 ( 16.7%) | 0 | 1 ( 8.3%) |
| 95% Exact CI | 0.0042, 0.6412 | 0.0000, 0.4593 | 0.0021, 0.3848 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.17 (-0.26, 0.59) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.17 (-0.45, 0.70) | | |
| Non-Responder, n(%) | 5 ( 83.3%) | 6 (100.0%) | 11 ( 91.7%) |
| Last participation date < Day 78 in RT Phase | 2 ( 33.3%) | 0 | 2 ( 16.7%) |
| Last participation date >= Day 78 and TSS at Week 12 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 12 | 3 ( 50.0%) | 2 ( 33.3%) | 5 ( 41.7%) |
| <50% reduction from baseline at Week 12 | 3 ( 50.0%) | 5 ( 83.3%) | 8 ( 66.7%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel; PP = Per-Protocol.

Data Extracted: CRF data: 25JUN2019

Table 2.2202: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Strata 4 | | | |
| Transfusion Dependence No and Baseline TSS >=18 | 5 | 8 | 13 |
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 0 | 0 | 0 |
| TSS > 0 at baseline | 5 (100.0%) | 8 (100.0%) | 13 (100.0%) |
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 5 | 8 | 13 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 0 | 0 |
| Responder, n(%) | 2 ( 40.0%) | 1 ( 12.5%) | 3 ( 23.1%) |
| 95% Exact CI | 0.0527, 0.8534 | 0.0032, 0.5265 | 0.0504, 0.5381 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.28 (-0.28, 0.83) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.28 (-0.29, 0.75) | | |
| Non-Responder, n(%) | 3 ( 60.0%) | 7 ( 87.5%) | 10 ( 76.9%) |
| Last participation date < Day 162 in RT Phase | 1 ( 20.0%) | 2 ( 25.0%) | 3 ( 23.1%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 24 | 1 ( 20.0%) | 2 ( 25.0%) | 3 ( 23.1%) |
| <50% reduction from baseline at Week 24 | 2 ( 40.0%) | 5 ( 62.5%) | 7 ( 53.8%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel; PP = Per-Protocol.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-tss24.sas V.03.05 Output file: t-sen-ppt-tss24.pdf 24AUG2023:16:38

Table 2.2202: Sensitivity Analysis of Response Rate in TSS at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 12 | | | |
| Subjects Evaluable at Week 12, n | 5 | 8 | 13 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 0 | 0 |
| Responder, n(%) | 2 ( 40.0%) | 1 ( 12.5%) | 3 ( 23.1%) |
| 95% Exact CI | 0.0527, 0.8534 | 0.0032, 0.5265 | 0.0504, 0.5381 |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.28 (-0.28, 0.83) | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.28 (-0.29, 0.75) | | |
| Non-Responder, n(%) | 3 ( 60.0%) | 7 ( 87.5%) | 10 ( 76.9%) |
| Last participation date < Day 78 in RT Phase | 0 | 2 ( 25.0%) | 2 ( 15.4%) |
| Last participation date >= Day 78 and TSS at Week 12 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 12 | 1 ( 20.0%) | 2 ( 25.0%) | 3 ( 23.1%) |
| <50% reduction from baseline at Week 12 | 3 ( 60.0%) | 5 ( 62.5%) | 8 ( 61.5%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
TSS rate analysis at one visit only include subjects with TSS >0 at baseline or with TSS =0 at baseline but with TSS > 0 or missing at that visit.
TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel; PP = Per-Protocol.

Data Extracted: CRF data: 25JUN2019

Table 2.1401: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Total Symptom Score Status | | | |
| Missing TSS at baseline (excluded from rate calculation) | 0 | 0 | 0 |
| TSS = 0 at baseline | 1 ( 1.5%) | 3 ( 7.7%) | 4 ( 3.8%) |
| TSS > 0 at baseline | 65 ( 98.5%) | 36 ( 92.3%) | 101 ( 96.2%) |
| Response Rate of Total Symptom Score at Week 4 | | | |
| Subjects Evaluable at Week 4, n | 66 | 38 | 104 |
| TSS = 0 at baseline and TSS >0 or missing at Week 4 | 1 ( 1.5%) | 2 ( 5.3%) | 3 ( 2.9%) |
| Responder, n(%) | 5 ( 7.6%) | 2 ( 5.3%) | 7 ( 6.7%) |
| 95% Exact CI | 0.0251, 0.1680 | 0.0064, 0.1775 | 0.0275, 0.1338 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.03 (-0.09, 0.15) | | |
| p-value | 0.60 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.02 (-0.08, 0.13) | | |
| p-value | 0.66 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.02 (-0.17, 0.22) | | |
| p-value | 1.00 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 0.69 (0.14, 3.41) | | |
| p-value [1] | 0.65 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 0.68 (0.12, 3.68) | | |
| p-value [1] | 0.65 | | |
| Unadjusted Absolute Risk Difference (95% CI) | 0.02 (-0.07, 0.12) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | 0.61 (0.14, 2.63) | | |
| p-value [2] | 0.51 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.1401: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Non-Responder, n(%) | 61 ( 92.4%) | 36 ( 94.7%) | 97 ( 93.3%) |
| Last participation date < Day 21 in RT Phase | 0 | 2 ( 5.3%) | 2 ( 1.9%) |
| Last participation date >= Day 21 and TSS at Week 4 not available | 0 | 0 | 0 |
| >0% increase from baseline at Week 4 | 27 ( 40.9%) | 22 ( 57.9%) | 49 ( 47.1%) |
| <50% reduction from baseline at Week 4 | 60 ( 90.9%) | 32 ( 84.2%) | 92 ( 88.5%) |
| Return Rate (%) | 100% | 95% | 98% |
| Number of Subjects in Risk | 66 | 37 | 103 |
| Return Rate in Risk (%) | 100% | 100% | 100% |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.1401: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 8 | | | |
| Subjects Evaluable at Week 8, n | 66 | 38 | 104 |
| TSS = 0 at baseline and TSS >0 or missing at Week 8 | 1 ( 1.5%) | 2 ( 5.3%) | 3 ( 2.9%) |
| Responder, n(%) | 12 ( 18.2%) | 3 ( 7.9%) | 15 ( 14.4%) |
| 95% Exact CI | 0.0976, 0.2961 | 0.0166, 0.2138 | 0.0830, 0.2267 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.08 (-0.06, 0.22) | | |
| p-value | 0.25 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.10 (-0.03, 0.23) | | |
| p-value | 0.13 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.10 (-0.10, 0.30) | | |
| p-value | 0.25 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 0.43 (0.13, 1.44) | | |
| p-value [1] | 0.17 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 0.39 (0.10, 1.47) | | |
| p-value [1] | 0.16 | | |
| Unadjusted Absolute Risk Difference (95% CI) | 0.10 (-0.02, 0.23) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | 0.50 (0.16, 1.59) | | |
| p-value [2] | 0.24 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.1401: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Non-Responder, n(%) | 54 ( 81.8%) | 35 ( 92.1%) | 89 ( 85.6%) |
| Last participation date < Day 50 in RT Phase | 5 ( 7.6%) | 3 ( 7.9%) | 8 ( 7.7%) |
| Last participation date >= Day 50 and TSS at Week 8 not available | 2 ( 3.0%) | 1 ( 2.6%) | 3 ( 2.9%) |
| >0% increase from baseline at Week 8 | 19 ( 28.8%) | 20 ( 52.6%) | 39 ( 37.5%) |
| <50% reduction from baseline at Week 8 | 46 ( 69.7%) | 29 ( 76.3%) | 75 ( 72.1%) |
| Return Rate (%) | 89% | 90% | 90% |
| Number of Subjects in Risk | 61 | 36 | 97 |
| Return Rate in Risk (%) | 97% | 97% | 97% |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.1401: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **Response Rate of Total Symptom Score at Week 12** | | | |
| Subjects Evaluable at Week 12, n | 65 | 38 | 103 |
| TSS = 0 at baseline and TSS >0 or missing at Week 12 | 0 | 2 ( 5.3%) | 2 ( 1.9%) |
| Responder, n(%) | 19 ( 29.2%) | 3 ( 7.9%) | 22 ( 21.4%) |
| 95% Exact CI | 0.1860, 0.4183 | 0.0166, 0.2138 | 0.1390, 0.3053 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.21 (0.05, 0.37) | | |
| p-value | 0.012 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.21 (0.07, 0.36) | | |
| p-value | 0.004 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.21 (0.01, 0.40) | | |
| p-value | 0.012 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 0.27 (0.09, 0.85) | | |
| p-value [1] | 0.026 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 0.21 (0.06, 0.76) | | |
| p-value [1] | 0.017 | | |
| Unadjusted Absolute Risk Difference (95% CI) | 0.21 (0.07, 0.35) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | 0.28 (0.09, 0.88) | | |
| p-value [2] | 0.029 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.1401: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Non-Responder, n(%) | 46 ( 70.8%) | 35 ( 92.1%) | 81 ( 78.6%) |
| Last participation date < Day 78 in RT Phase | 7 ( 10.8%) | 6 ( 15.8%) | 13 ( 12.6%) |
| Last participation date >= Day 78 and TSS at Week 12 not available | 2 ( 3.1%) | 0 | 2 ( 1.9%) |
| >0% increase from baseline at Week 12 | 18 ( 27.7%) | 17 ( 44.7%) | 35 ( 34.0%) |
| <50% reduction from baseline at Week 12 | 37 ( 56.9%) | 27 ( 71.1%) | 64 ( 62.1%) |
| Return Rate (%) | 86% | 85% | 86% |
| Number of Subjects in Risk | 59 | 33 | 92 |
| Return Rate in Risk (%) | 97% | 100% | 98% |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.1401: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 16 | | | |
| Subjects Evaluable at Week 16, n | 65 | 38 | 103 |
| TSS = 0 at baseline and TSS >0 or missing at Week 16 | 0 | 2 ( 5.3%) | 2 ( 1.9%) |
| Responder, n(%) | 19 ( 29.2%) | 2 ( 5.3%) | 21 ( 20.4%) |
| 95% Exact CI | 0.1860, 0.4183 | 0.0064, 0.1775 | 0.1309, 0.2946 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.25 (0.09, 0.41) | | |
| p-value | 0.002 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.24 (0.10, 0.38) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.24 (0.04, 0.43) | | |
| p-value | 0.004 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 0.18 (0.04, 0.73) | | |
| p-value [1] | 0.016 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 0.13 (0.03, 0.62) | | |
| p-value [1] | 0.010 | | |
| Unadjusted Absolute Risk Difference (95% CI) | 0.24 (0.11, 0.37) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | 0.17 (0.04, 0.69) | | |
| p-value [2] | 0.013 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.1401: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Non-Responder, n(%) | 46 ( 70.8%) | 36 ( 94.7%) | 82 ( 79.6%) |
| Last participation date < Day 106 in RT Phase | 10 ( 15.4%) | 7 ( 18.4%) | 17 ( 16.5%) |
| Last participation date >= Day 106 and TSS at Week 16 not available | 4 ( 6.2%) | 0 | 4 ( 3.9%) |
| >0% increase from baseline at Week 16 | 15 ( 23.1%) | 17 ( 44.7%) | 32 ( 31.1%) |
| <50% reduction from baseline at Week 16 | 32 ( 49.2%) | 27 ( 71.1%) | 59 ( 57.3%) |
| Return Rate (%) | 79% | 82% | 80% |
| Number of Subjects in Risk | 56 | 32 | 88 |
| Return Rate in Risk (%) | 93% | 100% | 95% |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.1401: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 20 | | | |
| Subjects Evaluable at Week 20, n | 65 | 38 | 103 |
| TSS = 0 at baseline and TSS >0 or missing at Week 20 | 0 | 2 ( 5.3%) | 2 ( 1.9%) |
| Responder, n(%) | 20 ( 30.8%) | 3 ( 7.9%) | 23 ( 22.3%) |
| 95% Exact CI | 0.1991, 0.4345 | 0.0166, 0.2138 | 0.1471, 0.3160 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.23 (0.07, 0.39) | | |
| p-value | 0.005 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.23 (0.08, 0.37) | | |
| p-value | 0.002 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.23 (0.03, 0.42) | | |
| p-value | 0.007 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 0.26 (0.08, 0.81) | | |
| p-value [1] | 0.020 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 0.19 (0.05, 0.70) | | |
| p-value [1] | 0.012 | | |
| Unadjusted Absolute Risk Difference (95% CI) | 0.23 (0.09, 0.37) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | 0.26 (0.08, 0.80) | | |
| p-value [2] | 0.019 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.1401: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Non-Responder, n(%) | 45 ( 69.2%) | 35 ( 92.1%) | 80 ( 77.7%) |
| Last participation date < Day 134 in RT Phase | 15 ( 23.1%) | 7 ( 18.4%) | 22 ( 21.4%) |
| Last participation date >= Day 134 and TSS at Week 20 not available | 1 ( 1.5%) | 0 | 1 ( 1.0%) |
| >0% increase from baseline at Week 20 | 17 ( 26.2%) | 17 ( 44.7%) | 34 ( 33.0%) |
| <50% reduction from baseline at Week 20 | 29 ( 44.6%) | 26 ( 68.4%) | 55 ( 53.4%) |
| Return Rate (%) | 76% | 82% | 78% |
| Number of Subjects in Risk | 51 | 32 | 83 |
| Return Rate in Risk (%) | 98% | 100% | 99% |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.1401: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Response Rate of Total Symptom Score at Week 24 | | | |
| Subjects Evaluable at Week 24, n | 65 | 38 | 103 |
| TSS = 0 at baseline and TSS >0 or missing at Week 24 | 0 | 2 ( 5.3%) | 2 ( 1.9%) |
| Responder, n(%) | 21 ( 32.3%) | 1 ( 2.6%) | 22 ( 21.4%) |
| 95% Exact CI | 0.2123, 0.4505 | 0.0007, 0.1381 | 0.1390, 0.3053 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.30 (0.15, 0.44) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.30 (0.17, 0.43) | | |
| p-value | <0.001 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.30 (0.10, 0.48) | | |
| p-value | <0.001 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 0.08 (0.01, 0.58) | | |
| p-value [1] | 0.012 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 0.06 (0.01, 0.44) | | |
| p-value [1] | 0.006 | | |
| Unadjusted Absolute Risk Difference (95% CI) | 0.30 (0.17, 0.42) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | 0.08 (0.01, 0.54) | | |
| p-value [2] | 0.010 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

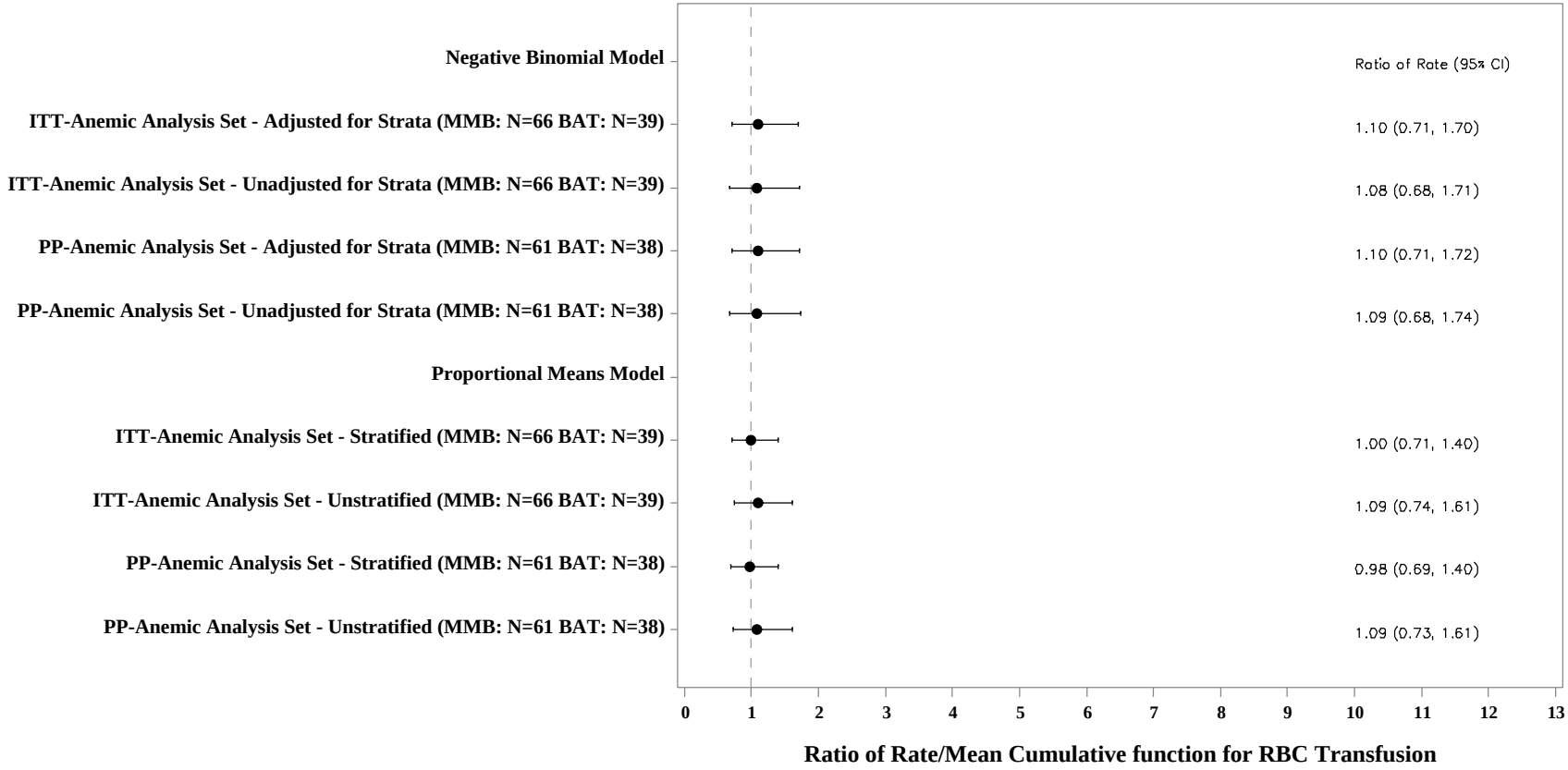
Table 2.1401: Analysis of Response Rate (improvement, 50% criterion) in TSS by Every 4 Weeks
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Non-Responder, n(%) | 44 ( 67.7%) | 37 ( 97.4%) | 81 ( 78.6%) |
| Last participation date < Day 162 in RT Phase | 18 ( 27.7%) | 7 ( 18.4%) | 25 ( 24.3%) |
| Last participation date >= Day 162 and TSS at Week 24 not available | 1 ( 1.5%) | 3 ( 7.9%) | 4 ( 3.9%) |
| >0% increase from baseline at Week 24 | 15 ( 23.1%) | 17 ( 44.7%) | 32 ( 31.1%) |
| <50% reduction from baseline at Week 24 | 25 ( 38.5%) | 26 ( 68.4%) | 51 ( 49.5%) |
| Return Rate (%) | 71% | 74% | 72% |
| Number of Subjects in Risk | 48 | 32 | 80 |
| Return Rate in Risk (%) | 98% | 91% | 95% |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification. TSS = Total Symptom Score, CMH = Cochran-Mantel-Haenszel.
Response rate analysis at each visit only includes subjects with TSS > 0 at baseline or (TSS = 0 at baseline and [TSS > 0 or missing]).
Subjects with TSS = 0 at baseline and = 0 at the visit are excluded from response rate analysis but are included in return rate calculations.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

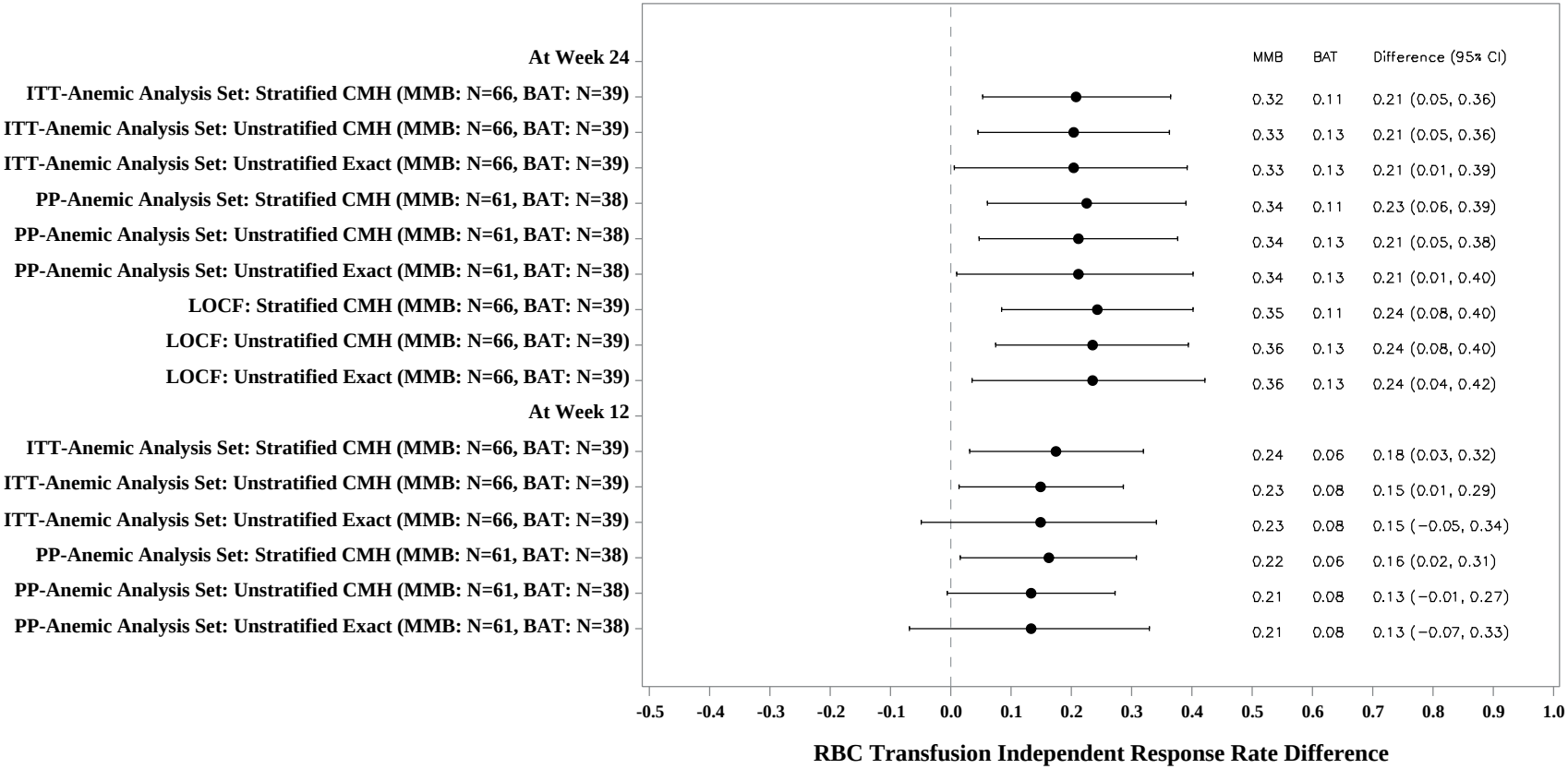
Figure 2.1401: Forest Plot of Primary and Sensitivity Analysis of Rate of RBC Transfusion
Randomized Treatment Phase
ITT-Anemic Analysis Set
All strata combined




ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Adjusted analysis for strata is used for negative binomial method. Stratified analysis is used for Proportional Means method.
To the left of the reference line favors MMB, to the right favors BAT.
RBC = Red Blood Cell; PP = Per-Protocol; CI = Confidence Interval.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/g-rbc-forest.sas V.03.05 Output file: g-rbc-forest.pdf 25AUG2023:13:36

Figure 2.1901: Forest Plot of Primary and Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set
All strata combined




ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
To the right of the reference line favors MMB, to the left favors BAT.
CMH = Cochran-Mantel-Haenszel; LOCF = Last Observation Carried Forward; PP=Per-Protocol; CI= Confidence Interval
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/g-rbc-ti-forest.sas V.03.05 Output file: g-rbc-ti-forest.pdf 25AUG2023:13:36

Table 2.3001: Analysis of RBC Transfusion Independence Response Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **All Strata Combined** | | | |
| **RBC Transfusion Independent Rate at Week 24** | | | |
| Responder, n(%) | 22 ( 33.3%) | 5 ( 12.8%) | 27 ( 25.7%) |
| 95% Exact CI | 0.2220, 0.4601 | 0.0430, 0.2743 | 0.1768, 0.3517 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.21 (0.05, 0.36) | | |
| p-value | 0.009 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.21 (0.05, 0.36) | | |
| p-value | 0.011 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.21 (0.01, 0.39) | | |
| p-value | 0.022 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 0.38 (0.16, 0.93) | | |
| p-value [1] | 0.035 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 0.29 (0.10, 0.86) | | |
| p-value [1] | 0.025 | | |
| Unadjusted Absolute Risk Difference (95% CI) | 0.21 (0.05, 0.36) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | 0.38 (0.17, 0.86) | | |
| p-value [2] | 0.021 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-tf-ti-gba-rr.sas V.03.05 Output file: t-rbcti24-gba.pdf 24AUG2023:16:58

Table 2.3001: Analysis of RBC Transfusion Independence Response Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Non-Responder, n(%) | 44 ( 66.7%) | 34 ( 87.2%) | 78 ( 74.3%) |
| Transfusion (except bleeding) in the last 12 weeks | 26 ( 39.4%) | 25 ( 64.1%) | 51 ( 48.6%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 21 ( 31.8%) | 18 ( 46.2%) | 39 ( 37.1%) |
| Last Participation date < Day 162 in RT phase | 18 ( 27.3%) | 7 ( 17.9%) | 25 ( 23.8%) |
| Other | 9 ( 13.6%) | 3 ( 7.7%) | 12 ( 11.4%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3001: Analysis of RBC Transfusion Independence Response Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **RBC Transfusion Independent Rate at Week 12** | | | |
| Responder, n(%) | 15 ( 22.7%) | 3 ( 7.7%) | 18 ( 17.1%) |
| 95% Exact CI | 0.1331, 0.3470 | 0.0162, 0.2087 | 0.1049, 0.2573 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.18 (0.03, 0.32) | | |
| p-value | 0.017 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | 0.15 (0.01, 0.29) | | |
| p-value | 0.031 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.15 (-0.05, 0.34) | | |
| p-value | 0.062 | | |
| Unadjusted Inverse Relative Risk (95% CI) | 0.34 (0.10, 1.10) | | |
| p-value [1] | 0.071 | | |
| Unadjusted Inverse Odds Ratio (95% CI) | 0.28 (0.08, 1.05) | | |
| p-value [1] | 0.059 | | |
| Unadjusted Absolute Risk Difference (95% CI) | 0.15 (0.02, 0.28) | | |
| Adjusted Inverse Relative Risk (95% CI) [2] | 0.29 (0.10, 0.85) | | |
| p-value [2] | 0.024 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3001: Analysis of RBC Transfusion Independence Response Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Non-Responder, n(%) | 51 ( 77.3%) | 36 ( 92.3%) | 87 ( 82.9%) |
| Transfusion (except bleeding) in the last 12 weeks | 42 ( 63.6%) | 28 ( 71.8%) | 70 ( 66.7%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 36 ( 54.5%) | 20 ( 51.3%) | 56 ( 53.3%) |
| Last Participation date < Day 78 in RT phase | 7 ( 10.6%) | 6 ( 15.4%) | 13 ( 12.4%) |
| Other | 20 ( 30.3%) | 5 ( 12.8%) | 25 ( 23.8%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3001: Analysis of RBC Transfusion Independence Response Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Baseline TSS <18 | 35 | 14 | 49 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 14 ( 40.0%) | 0 | 14 ( 28.6%) |
| 95% Exact CI | 0.2387, 0.5789 | 0.0000, 0.2316 | 0.1658, 0.4326 |
| Proportion Difference (95% CI) | 0.40 (0.21, 0.59) | | |
| Proportion Difference using Exact Method (95% CI) | 0.40 (0.09, 0.67) | | |
| Non-Responder, n(%) | 21 ( 60.0%) | 14 (100.0%) | 35 ( 71.4%) |
| Transfusion (except bleeding) in the last 12 weeks | 15 ( 42.9%) | 11 ( 78.6%) | 26 ( 53.1%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 12 ( 34.3%) | 6 ( 42.9%) | 18 ( 36.7%) |
| Last Participation date < Day 162 in RT phase | 6 ( 17.1%) | 2 ( 14.3%) | 8 ( 16.3%) |
| Other | 7 ( 20.0%) | 2 ( 14.3%) | 9 ( 18.4%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3001: Analysis of RBC Transfusion Independence Response Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **RBC Transfusion Independent Rate at Week 12** | | | |
| Responder, n(%) | 7 ( 20.0%) | 0 | 7 ( 14.3%) |
| 95% Exact CI | 0.0844, 0.3694 | 0.0000, 0.2316 | 0.0594, 0.2724 |
| Proportion Difference (95% CI) | 0.20 (0.03, 0.37) | | |
| Proportion Difference using Exact Method (95% CI) | 0.20 (-0.12, 0.49) | | |
| Non-Responder, n(%) | 28 ( 80.0%) | 14 (100.0%) | 42 ( 85.7%) |
| Transfusion (except bleeding) in the last 12 weeks | 25 ( 71.4%) | 12 ( 85.7%) | 37 ( 75.5%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 22 ( 62.9%) | 9 ( 64.3%) | 31 ( 63.3%) |
| Last Participation date < Day 78 in RT phase | 1 ( 2.9%) | 2 ( 14.3%) | 3 ( 6.1%) |
| Other | 13 ( 37.1%) | 3 ( 21.4%) | 16 ( 32.7%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3001: Analysis of RBC Transfusion Independence Response Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Baseline TSS >=18 | 17 | 11 | 28 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 3 ( 17.6%) | 1 ( 9.1%) | 4 ( 14.3%) |
| 95% Exact CI | 0.0380, 0.4343 | 0.0023, 0.4128 | 0.0403, 0.3267 |
| Proportion Difference (95% CI) | 0.09 (-0.20, 0.37) | | |
| Proportion Difference using Exact Method (95% CI) | 0.09 (-0.28, 0.44) | | |
| Non-Responder, n(%) | 14 ( 82.4%) | 10 ( 90.9%) | 24 ( 85.7%) |
| Transfusion (except bleeding) in the last 12 weeks | 8 ( 47.1%) | 8 ( 72.7%) | 16 ( 57.1%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 7 ( 41.2%) | 7 ( 63.6%) | 14 ( 50.0%) |
| Last Participation date < Day 162 in RT phase | 6 ( 35.3%) | 2 ( 18.2%) | 8 ( 28.6%) |
| Other | 2 ( 11.8%) | 1 ( 9.1%) | 3 ( 10.7%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3001: Analysis of RBC Transfusion Independence Response Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **RBC Transfusion Independent Rate at Week 12** | | | |
| Responder, n(%) | 3 ( 17.6%) | 0 | 3 ( 10.7%) |
| 95% Exact CI | 0.0380, 0.4343 | 0.0000, 0.2849 | 0.0227, 0.2823 |
| Proportion Difference (95% CI) | 0.18 (-0.05, 0.41) | | |
| Proportion Difference using Exact Method (95% CI) | 0.18 (-0.20, 0.52) | | |
| Non-Responder, n(%) | 14 ( 82.4%) | 11 (100.0%) | 25 ( 89.3%) |
| Transfusion (except bleeding) in the last 12 weeks | 11 ( 64.7%) | 9 ( 81.8%) | 20 ( 71.4%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 11 ( 64.7%) | 8 ( 72.7%) | 19 ( 67.9%) |
| Last Participation date < Day 78 in RT phase | 3 ( 17.6%) | 2 ( 18.2%) | 5 ( 17.9%) |
| Other | 7 ( 41.2%) | 1 ( 9.1%) | 8 ( 28.6%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-tf-ti-gba-rr.sas V.03.05 Output file: t-rbcti24-gba.pdf 24AUG2023:16:58

Table 2.3001: Analysis of RBC Transfusion Independence Response Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence No and Baseline TSS <18 | 7 | 6 | 13 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 4 ( 57.1%) | 2 ( 33.3%) | 6 ( 46.2%) |
| 95% Exact CI | 0.1841, 0.9010 | 0.0433, 0.7772 | 0.1922, 0.7487 |
| Proportion Difference (95% CI) | 0.24 (-0.34, 0.82) | | |
| Proportion Difference using Exact Method (95% CI) | 0.24 (-0.34, 0.71) | | |
| Non-Responder, n(%) | 3 ( 42.9%) | 4 ( 66.7%) | 7 ( 53.8%) |
| Transfusion (except bleeding) in the last 12 weeks | 0 | 2 ( 33.3%) | 2 ( 15.4%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 0 | 3 ( 50.0%) | 3 ( 23.1%) |
| Last Participation date < Day 162 in RT phase | 3 ( 42.9%) | 1 ( 16.7%) | 4 ( 30.8%) |
| Other | 0 | 0 | 0 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3001: Analysis of RBC Transfusion Independence Response Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **RBC Transfusion Independent Rate at Week 12** | | | |
| Responder, n(%) | 3 ( 42.9%) | 1 ( 16.7%) | 4 ( 30.8%) |
| 95% Exact CI | 0.0990, 0.8159 | 0.0042, 0.6412 | 0.0909, 0.6143 |
| Proportion Difference (95% CI) | 0.26 (-0.27, 0.80) | | |
| Proportion Difference using Exact Method (95% CI) | 0.26 (-0.29, 0.72) | | |
| Non-Responder, n(%) | 4 ( 57.1%) | 5 ( 83.3%) | 9 ( 69.2%) |
| Transfusion (except bleeding) in the last 12 weeks | 2 ( 28.6%) | 3 ( 50.0%) | 5 ( 38.5%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 1 ( 14.3%) | 2 ( 33.3%) | 3 ( 23.1%) |
| Last Participation date < Day 78 in RT phase | 2 ( 28.6%) | 0 | 2 ( 15.4%) |
| Other | 0 | 1 ( 16.7%) | 1 (  7.7%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3001: Analysis of RBC Transfusion Independence Response Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Baseline TSS >=18 | 7 | 8 | 15 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 1 ( 14.3%) | 2 ( 25.0%) | 3 ( 20.0%) |
| 95% Exact CI | 0.0036, 0.5787 | 0.0319, 0.6509 | 0.0433, 0.4809 |
| Proportion Difference (95% CI) | -0.11 (-0.56, 0.35) | | |
| Proportion Difference using Exact Method (95% CI) | -0.11 (-0.58, 0.36) | | |
| Non-Responder, n(%) | 6 ( 85.7%) | 6 ( 75.0%) | 12 ( 80.0%) |
| Transfusion (except bleeding) in the last 12 weeks | 3 ( 42.9%) | 4 ( 50.0%) | 7 ( 46.7%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 2 ( 28.6%) | 2 ( 25.0%) | 4 ( 26.7%) |
| Last Participation date < Day 162 in RT phase | 3 ( 42.9%) | 2 ( 25.0%) | 5 ( 33.3%) |
| Other | 0 | 0 | 0 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3001: Analysis of RBC Transfusion Independence Response Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **RBC Transfusion Independent Rate at Week 12** | | | |
| Responder, n(%) | 2 ( 28.6%) | 2 ( 25.0%) | 4 ( 26.7%) |
| 95% Exact CI | 0.0367, 0.7096 | 0.0319, 0.6509 | 0.0779, 0.5510 |
| Proportion Difference (95% CI) | 0.04 (-0.46, 0.53) | | |
| Proportion Difference using Exact Method (95% CI) | 0.04 (-0.47, 0.51) | | |
| Non-Responder, n(%) | 5 ( 71.4%) | 6 ( 75.0%) | 11 ( 73.3%) |
| Transfusion (except bleeding) in the last 12 weeks | 4 ( 57.1%) | 4 ( 50.0%) | 8 ( 53.3%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 2 ( 28.6%) | 1 ( 12.5%) | 3 ( 20.0%) |
| Last Participation date < Day 78 in RT phase | 1 ( 14.3%) | 2 ( 25.0%) | 3 ( 20.0%) |
| Other | 0 | 0 | 0 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-tf-ti-gba-rr.sas V.03.05 Output file: t-rbcti24-gba.pdf 24AUG2023:16:58

Table 2.3601: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24--LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **All Strata Combined** | | | |
| **RBC Transfusion Independent Rate at Week 24** | | | |
| Responder, n(%) | 24 ( 36.4%) | 5 ( 12.8%) | 29 ( 27.6%) |
| 95% Exact CI | 0.2487, 0.4913 | 0.0430, 0.2743 | 0.1934, 0.3720 |
| Proportion Difference - Stratified CMH Method(95% CI) | 0.24(0.08, 0.40) | | |
| P-value | 0.003 | | |
| Proportion Difference - Unstratified Method (95% CI) | 0.24(0.08, 0.40) | | |
| P-value | 0.004 | | |
| Proportion Difference - Unstratified Exact Method(95% CI) | 0.24(0.04, 0.42) | | |
| P-value | 0.012 | | |
| Non-Responder, n(%) | 42 ( 63.6%) | 34 ( 87.2%) | 76 ( 72.4%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.3601: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24--LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Baseline TSS <18 | 35 | 14 | 49 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 15 ( 42.9%) | 0 | 15 ( 30.6%) |
| 95% Exact CI | 0.2632, 0.6065 | 0.0000, 0.2316 | 0.1825, 0.4542 |
| Proportion Difference (95% CI) | 0.43(0.24, 0.62) | | |
| Proportion Difference using Exact Method(95% CI) | 0.43(0.12, 0.71) | | |
| Non-Responder, n(%) | 20 ( 57.1%) | 14 (100.0%) | 34 ( 69.4%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.3601: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24--LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Baseline TSS >=18 | 17 | 11 | 28 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 3 ( 17.6%) | 1 ( 9.1%) | 4 ( 14.3%) |
| 95% Exact CI | 0.0380, 0.4343 | 0.0023, 0.4128 | 0.0403, 0.3267 |
| Proportion Difference (95% CI) | 0.09(-0.20, 0.37) | | |
| Proportion Difference using Exact Method(95% CI) | 0.09(-0.28, 0.44) | | |
| Non-Responder, n(%) | 14 ( 82.4%) | 10 ( 90.9%) | 24 ( 85.7%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.3601: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24--LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **Stratum 3** | | | |
| Transfusion Dependence No and Baseline TSS <18 | 7 | 6 | 13 |
| **RBC Transfusion Independent Rate at Week 24** | | | |
| Responder, n(%) | 4 ( 57.1%) | 2 ( 33.3%) | 6 ( 46.2%) |
| 95% Exact CI | 0.1841, 0.9010 | 0.0433, 0.7772 | 0.1922, 0.7487 |
| Proportion Difference (95% CI) | 0.24(-0.34, 0.82) | | |
| Proportion Difference using Exact Method(95% CI) | 0.24(-0.34, 0.71) | | |
| Non-Responder, n(%) | 3 ( 42.9%) | 4 ( 66.7%) | 7 ( 53.8%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.3601: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24--LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Baseline TSS >=18 | 7 | 8 | 15 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 2 ( 28.6%) | 2 ( 25.0%) | 4 ( 26.7%) |
| 95% Exact CI | 0.0367, 0.7096 | 0.0319, 0.6509 | 0.0779, 0.5510 |
| Proportion Difference (95% CI) | 0.04(-0.46, 0.53) | | |
| Proportion Difference using Exact Method(95% CI) | 0.04(-0.47, 0.51) | | |
| Non-Responder, n(%) | 5 ( 71.4%) | 6 ( 75.0%) | 11 ( 73.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.3602: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **All Strata Combined** | | | |
| **RBC Transfusion Independent Rate at Week 24** | | | |
| Responder, n(%) | 21 ( 34.4%) | 5 ( 13.2%) | 26 ( 26.3%) |
| 95% Exact CI | 0.2273, 0.4769 | 0.0441, 0.2809 | 0.1793, 0.3607 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.23 (0.06, 0.39) | | |
| p-value | 0.007 | | |
| Proportion Difference - Unstratified Method (95% CI) | 0.21 (0.05, 0.38) | | |
| p-value | 0.011 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.21 (0.01, 0.40) | | |
| p-value | 0.021 | | |
| Non-Responder, n(%) | 40 ( 65.6%) | 33 ( 86.8%) | 73 ( 73.7%) |
| Transfusion (except bleeding) in the last 12 weeks | 24 ( 39.3%) | 24 ( 63.2%) | 48 ( 48.5%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 19 ( 31.1%) | 17 ( 44.7%) | 36 ( 36.4%) |
| Last Participation date < Day 162 in RT phase | 16 ( 26.2%) | 7 ( 18.4%) | 23 ( 23.2%) |
| Other | 8 ( 13.1%) | 2 ( 5.3%) | 10 ( 10.1%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-tf-ti.sas V.03.05 Output file: t-sen-ppt-rbcti24.pdf 24AUG2023:16:39

Table 2.3602: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **RBC Transfusion Independent Rate at Week 12** | | | |
| Responder, n(%) | 13 ( 21.3%) | 3 ( 7.9%) | 16 ( 16.2%) |
| 95% Exact CI | 0.1186, 0.3368 | 0.0166, 0.2138 | 0.0953, 0.2491 |
| Proportion Difference - Stratified CMH Method (95% CI) | 0.16 (0.02, 0.31) | | |
| p-value | 0.030 | | |
| Proportion Difference - Unstratified Method (95% CI) | 0.13 (-0.01, 0.27) | | |
| p-value | 0.059 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | 0.13 (-0.07, 0.33) | | |
| p-value | 0.097 | | |
| Non-Responder, n(%) | 48 ( 78.7%) | 35 ( 92.1%) | 83 ( 83.8%) |
| Transfusion (except bleeding) in the last 12 weeks | 40 ( 65.6%) | 27 ( 71.1%) | 67 ( 67.7%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 34 ( 55.7%) | 19 ( 50.0%) | 53 ( 53.5%) |
| Last Participation date < Day 78 in RT phase | 6 ( 9.8%) | 6 ( 15.8%) | 12 ( 12.1%) |
| Other | 18 ( 29.5%) | 4 ( 10.5%) | 22 ( 22.2%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.3602: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Baseline TSS <18 | 32 | 14 | 46 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 13 ( 40.6%) | 0 | 13 ( 28.3%) |
| 95% Exact CI | 0.2370, 0.5936 | 0.0000, 0.2316 | 0.1599, 0.4346 |
| Proportion Difference (95% CI) | 0.41 (0.21, 0.60) | | |
| Proportion Difference using Exact Method (95% CI) | 0.41 (0.10, 0.68) | | |
| Non-Responder, n(%) | 19 ( 59.4%) | 14 (100.0%) | 33 ( 71.7%) |
| Transfusion (except bleeding) in the last 12 weeks | 13 ( 40.6%) | 11 ( 78.6%) | 24 ( 52.2%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 10 ( 31.3%) | 6 ( 42.9%) | 16 ( 34.8%) |
| Last Participation date < Day 162 in RT phase | 6 ( 18.8%) | 2 ( 14.3%) | 8 ( 17.4%) |
| Other | 6 ( 18.8%) | 2 ( 14.3%) | 8 ( 17.4%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.3602: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **RBC Transfusion Independent Rate at Week 12** | | | |
| Responder, n(%) | 6 ( 18.8%) | 0 | 6 ( 13.0%) |
| 95% Exact CI | 0.0721, 0.3644 | 0.0000, 0.2316 | 0.0494, 0.2626 |
| Proportion Difference (95% CI) | 0.19 (0.02, 0.36) | | |
| Proportion Difference using Exact Method (95% CI) | 0.19 (-0.13, 0.49) | | |
| Non-Responder, n(%) | 26 ( 81.3%) | 14 (100.0%) | 40 ( 87.0%) |
| Transfusion (except bleeding) in the last 12 weeks | 23 ( 71.9%) | 12 ( 85.7%) | 35 ( 76.1%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 20 ( 62.5%) | 9 ( 64.3%) | 29 ( 63.0%) |
| Last Participation date < Day 78 in RT phase | 1 ( 3.1%) | 2 ( 14.3%) | 3 ( 6.5%) |
| Other | 11 ( 34.4%) | 3 ( 21.4%) | 14 ( 30.4%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.3602: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Baseline TSS >=18 | 17 | 10 | 27 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 3 ( 17.6%) | 1 ( 10.0%) | 4 ( 14.8%) |
| 95% Exact CI | 0.0380, 0.4343 | 0.0025, 0.4450 | 0.0419, 0.3373 |
| Proportion Difference (95% CI) | 0.08 (-0.22, 0.37) | | |
| Proportion Difference using Exact Method (95% CI) | 0.08 (-0.30, 0.44) | | |
| Non-Responder, n(%) | 14 ( 82.4%) | 9 ( 90.0%) | 23 ( 85.2%) |
| Transfusion (except bleeding) in the last 12 weeks | 8 ( 47.1%) | 7 ( 70.0%) | 15 ( 55.6%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 7 ( 41.2%) | 6 ( 60.0%) | 13 ( 48.1%) |
| Last Participation date < Day 162 in RT phase | 6 ( 35.3%) | 2 ( 20.0%) | 8 ( 29.6%) |
| Other | 2 ( 11.8%) | 0 | 2 ( 7.4%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.3602: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **RBC Transfusion Independent Rate at Week 12** | | | |
| Responder, n(%) | 3 ( 17.6%) | 0 | 3 ( 11.1%) |
| 95% Exact CI | 0.0380, 0.4343 | 0.0000, 0.3085 | 0.0235, 0.2916 |
| Proportion Difference (95% CI) | 0.18 (-0.06, 0.41) | | |
| Proportion Difference using Exact Method (95% CI) | 0.18 (-0.21, 0.53) | | |
| Non-Responder, n(%) | 14 ( 82.4%) | 10 (100.0%) | 24 ( 88.9%) |
| Transfusion (except bleeding) in the last 12 weeks | 11 ( 64.7%) | 8 ( 80.0%) | 19 ( 70.4%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 11 ( 64.7%) | 7 ( 70.0%) | 18 ( 66.7%) |
| Last Participation date < Day 78 in RT phase | 3 ( 17.6%) | 2 ( 20.0%) | 5 ( 18.5%) |
| Other | 7 ( 41.2%) | 0 | 7 ( 25.9%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.3602: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence No and Baseline TSS <18 | 7 | 6 | 13 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 4 ( 57.1%) | 2 ( 33.3%) | 6 ( 46.2%) |
| 95% Exact CI | 0.1841, 0.9010 | 0.0433, 0.7772 | 0.1922, 0.7487 |
| Proportion Difference (95% CI) | 0.24 (-0.34, 0.82) | | |
| Proportion Difference using Exact Method (95% CI) | 0.24 (-0.34, 0.71) | | |
| Non-Responder, n(%) | 3 ( 42.9%) | 4 ( 66.7%) | 7 ( 53.8%) |
| Transfusion (except bleeding) in the last 12 weeks | 0 | 2 ( 33.3%) | 2 ( 15.4%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 0 | 3 ( 50.0%) | 3 ( 23.1%) |
| Last Participation date < Day 162 in RT phase | 3 ( 42.9%) | 1 ( 16.7%) | 4 ( 30.8%) |
| Other | 0 | 0 | 0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.3602: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **RBC Transfusion Independent Rate at Week 12** | | | |
| Responder, n(%) | 3 ( 42.9%) | 1 ( 16.7%) | 4 ( 30.8%) |
| 95% Exact CI | 0.0990, 0.8159 | 0.0042, 0.6412 | 0.0909, 0.6143 |
| Proportion Difference (95% CI) | 0.26 (-0.27, 0.80) | | |
| Proportion Difference using Exact Method (95% CI) | 0.26 (-0.29, 0.72) | | |
| Non-Responder, n(%) | 4 ( 57.1%) | 5 ( 83.3%) | 9 ( 69.2%) |
| Transfusion (except bleeding) in the last 12 weeks | 2 ( 28.6%) | 3 ( 50.0%) | 5 ( 38.5%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 1 ( 14.3%) | 2 ( 33.3%) | 3 ( 23.1%) |
| Last Participation date < Day 78 in RT phase | 2 ( 28.6%) | 0 | 2 ( 15.4%) |
| Other | 0 | 1 ( 16.7%) | 1 (  7.7%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.3602: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Baseline TSS >=18 | 5 | 8 | 13 |
| RBC Transfusion Independent Rate at Week 24 | | | |
| Responder, n(%) | 1 ( 20.0%) | 2 ( 25.0%) | 3 ( 23.1%) |
| 95% Exact CI | 0.0051, 0.7164 | 0.0319, 0.6509 | 0.0504, 0.5381 |
| Proportion Difference (95% CI) | -0.05 (-0.59, 0.49) | | |
| Proportion Difference using Exact Method (95% CI) | -0.05 (-0.57, 0.50) | | |
| Non-Responder, n(%) | 4 ( 80.0%) | 6 ( 75.0%) | 10 ( 76.9%) |
| Transfusion (except bleeding) in the last 12 weeks | 3 ( 60.0%) | 4 ( 50.0%) | 7 ( 53.8%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 2 ( 40.0%) | 2 ( 25.0%) | 4 ( 30.8%) |
| Last Participation date < Day 162 in RT phase | 1 ( 20.0%) | 2 ( 25.0%) | 3 ( 23.1%) |
| Other | 0 | 0 | 0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.3602: Sensitivity Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **RBC Transfusion Independent Rate at Week 12** | | | |
| Responder, n(%) | 1 ( 20.0%) | 2 ( 25.0%) | 3 ( 23.1%) |
| 95% Exact CI | 0.0051, 0.7164 | 0.0319, 0.6509 | 0.0504, 0.5381 |
| Proportion Difference (95% CI) | -0.05 (-0.59, 0.49) | | |
| Proportion Difference using Exact Method (95% CI) | -0.05 (-0.57, 0.50) | | |
| Non-Responder, n(%) | 4 ( 80.0%) | 6 ( 75.0%) | 10 ( 76.9%) |
| Transfusion (except bleeding) in the last 12 weeks | 4 ( 80.0%) | 4 ( 50.0%) | 8 ( 61.5%) |
| Any Hgb assessment < 8g/dL in the last 12 weeks | 2 ( 40.0%) | 1 ( 12.5%) | 3 ( 23.1%) |
| Last Participation date < Day 78 in RT phase | 0 | 2 ( 25.0%) | 2 ( 15.4%) |
| Other | 0 | 0 | 0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.2701: Sensitivity Analysis of Rate of RBC Transfusion
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **All Strata Combined** | | | |
| RBC transfusion Rate in RT phase (units/month) | | | |
| N | 61 | 38 | 99 |
| Mean (SD) | 2.1 (2.10) | 2.0 (2.00) | 2.1 (2.05) |
| Median | 1.7 | 1.4 | 1.4 |
| Q1, Q3 | 0.4, 3.3 | 0.5, 2.9 | 0.4, 3.1 |
| Min, Max | 0.0, 8.2 | 0.0, 7.6 | 0.0, 8.2 |
| Negative Binomial Model | | | |
| Adjusted for Strata | | | |
| Rate of RBC transfusion with 95% CI (units/month) | 1.62 (1.19, 2.22) | 1.47 (1.02, 2.12) | |
| Ratio of Rate for RBC Transfusion with 95% CI | 1.10 (0.71, 1.72) | - | - |
| p-value | 0.66 | - | - |
| Unadjusted for Strata | | | |
| Rate of RBC transfusion with 95% CI (units/month) | 2.10 (1.58, 2.80) | 1.93 (1.33, 2.79) | |
| Ratio of Rate for RBC Transfusion with 95% CI | 1.09 (0.68, 1.74) | - | - |
| p-value | 0.72 | - | - |
| Proportional Means Model - Supportive Analysis | | | |
| Stratified | | | |
| Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI | 0.98 (0.69, 1.40) | - | - |
| p-value | 0.91 | - | - |
| Unstratified | | | |
| Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI | 1.09 (0.73, 1.61) | - | - |
| p-value | 0.67 | - | - |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.2701: Sensitivity Analysis of Rate of RBC Transfusion
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **Total number of RBC transfusion unit in RT phase** | | | |
| N | 61 | 38 | 99 |
| Mean (SD) | 9.6 (9.95) | 8.7 (8.44) | 9.3 (9.36) |
| Median | 6.0 | 6.5 | 6.0 |
| Q1, Q3 | 2.0, 13.0 | 3.0, 13.0 | 2.0, 13.0 |
| Min, Max | 0.0, 38.0 | 0.0, 40.0 | 0.0, 40.0 |
| **Duration of the RT phase (months)** | | | |
| N | 61 | 38 | 99 |
| Mean (SD) | 4.93 (1.366) | 4.88 (1.556) | 4.91 (1.434) |
| Median | 5.55 | 5.55 | 5.55 |
| Q1, Q3 | 5.29, 5.68 | 5.49, 5.62 | 5.32, 5.65 |
| Min, Max | 0.92, 5.78 | 0.43, 5.78 | 0.43, 5.78 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-sen-rbc24.sas V.03.05 Output file: t-sen-rbc24.pdf 24AUG2023:16:38

Table 2.2701: Sensitivity Analysis of Rate of RBC Transfusion
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence at Baseline = 'Yes' and TSS < 18 at Baseline | | | |
| RBC transfusion Rate in RT phase (units/month) | | | |
| N | 32 | 14 | 46 |
| Mean (SD) | 2.0 (2.09) | 2.0 (1.50) | 2.0 (1.91) |
| Median | 1.0 | 1.3 | 1.2 |
| Q1, Q3 | 0.4, 3.3 | 0.9, 3.0 | 0.5, 3.1 |
| Min, Max | 0.0, 6.8 | 0.0, 5.0 | 0.0, 6.8 |
| Negative Binomial Model | | | |
| Rate of RBC transfusion with 95% CI (units/month) | 1.99 (1.39, 2.85) | 2.03 (1.17, 3.51) | |
| Ratio of Rate for RBC Transfusion with 95% CI | 0.98 (0.51, 1.89) | - | - |
| Proportional Means Model - Supportive Analysis | | | |
| Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI | 0.98 (0.60, 1.62) | - | - |
| Total number of RBC transfusion in stratum 1 in RT phase | | | |
| N | 32 | 14 | 46 |
| Mean (SD) | 10.2 (11.08) | 9.8 (7.06) | 10.1 (9.95) |
| Median | 6.0 | 7.5 | 6.5 |
| Q1, Q3 | 2.0, 15.5 | 5.0, 16.0 | 2.0, 16.0 |
| Min, Max | 0.0, 38.0 | 0.0, 25.0 | 0.0, 38.0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.2701: Sensitivity Analysis of Rate of RBC Transfusion
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Duration of the RT phase (months) in stratum 1 | | | |
| N | 32 | 14 | 46 |
| Mean (SD) | 5.24 (0.888) | 4.96 (1.690) | 5.15 (1.177) |
| Median | 5.55 | 5.55 | 5.55 |
| Q1, Q3 | 5.47, 5.72 | 5.49, 5.75 | 5.49, 5.72 |
| Min, Max | 2.14, 5.78 | 0.43, 5.78 | 0.43, 5.78 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.2701: Sensitivity Analysis of Rate of RBC Transfusion
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence at Baseline = 'Yes' and TSS >= 18 at Baseline | | | |
| RBC transfusion Rate in RT phase (units/month) | | | |
| N | 17 | 10 | 27 |
| Mean (SD) | 2.9 (2.47) | 3.7 (2.52) | 3.2 (2.47) |
| Median | 2.2 | 2.9 | 2.5 |
| Q1, Q3 | 0.7, 4.5 | 1.4, 6.2 | 1.1, 5.8 |
| Min, Max | 0.0, 8.2 | 1.0, 7.6 | 0.0, 8.2 |
| Negative Binomial Model | | | |
| Rate of RBC transfusion with 95% CI (units/month) | 2.79 (1.79, 4.35) | 3.54 (1.99, 6.30) | |
| Ratio of Rate for RBC Transfusion with 95% CI | 0.79 (0.38, 1.63) | - | - |
| Proportional Means Model - Supportive Analysis | | | |
| Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI | 0.78 (0.43, 1.41) | - | - |
| Total number of RBC transfusion in stratum 2 in RT phase | | | |
| N | 17 | 10 | 27 |
| Mean (SD) | 11.2 (9.78) | 14.4 (10.71) | 12.4 (10.05) |
| Median | 9.0 | 10.5 | 10.0 |
| Q1, Q3 | 4.0, 16.0 | 8.0, 18.0 | 5.0, 17.0 |
| Min, Max | 0.0, 32.0 | 5.0, 40.0 | 0.0, 40.0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.2701: Sensitivity Analysis of Rate of RBC Transfusion
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Duration of the RT phase (months) in stratum 2 | | | |
| N | 17 | 10 | 27 |
| Mean (SD) | 4.64 (1.641) | 4.68 (1.849) | 4.66 (1.685) |
| Median | 5.52 | 5.55 | 5.55 |
| Q1, Q3 | 4.67, 5.55 | 5.32, 5.55 | 4.67, 5.55 |
| Min, Max | 0.92, 5.78 | 0.66, 5.78 | 0.66, 5.78 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-sen-rbc24.sas V.03.05 Output file: t-sen-rbc24.pdf 24AUG2023:16:38

Table 2.2701: Sensitivity Analysis of Rate of RBC Transfusion
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **Stratum 3** | | | |
| Transfusion Dependence at Baseline = 'No' and TSS < 18 at Baseline | | | |
| RBC transfusion Rate in RT phase (units/month) | | | |
| N | 7 | 6 | 13 |
| Mean (SD) | 1.2 (1.23) | 0.5 (0.66) | 0.9 (1.04) |
| Median | 1.0 | 0.2 | 0.4 |
| Q1, Q3 | 0.0, 2.1 | 0.0, 0.9 | 0.0, 1.6 |
| Min, Max | 0.0, 3.1 | 0.0, 1.6 | 0.0, 3.1 |
| Negative Binomial Model | | | |
| Rate of RBC transfusion with 95% CI (units/month) | 1.12 (0.35, 3.58) | 0.49 (0.14, 1.66) | |
| Ratio of Rate for RBC Transfusion with 95% CI | 2.29 (0.42, 12.44) | - | - |
| Proportional Means Model - Supportive Analysis | | | |
| Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI | 1.46 (0.39, 5.49) | - | - |
| Total number of RBC transfusion in stratum 3 in RT phase | | | |
| N | 7 | 6 | 13 |
| Mean (SD) | 3.0 (3.70) | 2.7 (3.67) | 2.8 (3.53) |
| Median | 2.0 | 1.0 | 2.0 |
| Q1, Q3 | 0.0, 6.0 | 0.0, 5.0 | 0.0, 5.0 |
| Min, Max | 0.0, 10.0 | 0.0, 9.0 | 0.0, 10.0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.2701: Sensitivity Analysis of Rate of RBC Transfusion
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Duration of the RT phase (months) in stratum 3 | | | |
| N | 7 | 6 | 13 |
| Mean (SD) | 3.96 (2.220) | 5.21 (0.899) | 4.54 (1.796) |
| Median | 5.49 | 5.55 | 5.55 |
| Q1, Q3 | 1.02, 5.72 | 5.49, 5.59 | 3.38, 5.59 |
| Min, Max | 0.95, 5.78 | 3.38, 5.72 | 0.95, 5.78 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.2701: Sensitivity Analysis of Rate of RBC Transfusion
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **Stratum 4** | | | |
| Transfusion Dependence at Baseline = 'No' and TSS >= 18 at Baseline | | | |
| RBC transfusion Rate in RT phase (units/month) | | | |
| N | 5 | 8 | 13 |
| Mean (SD) | 1.8 (1.08) | 0.9 (1.05) | 1.3 (1.10) |
| Median | 1.9 | 0.5 | 1.8 |
| Q1, Q3 | 1.9, 2.2 | 0.0, 2.1 | 0.0, 2.2 |
| Min, Max | 0.0, 2.9 | 0.0, 2.4 | 0.0, 2.9 |
| Negative Binomial Model | | | |
| Rate of RBC transfusion with 95% CI (units/month) | 1.78 (0.64, 4.93) | 0.93 (0.40, 2.18) | |
| Ratio of Rate for RBC Transfusion with 95% CI | 1.92 (0.51, 7.23) | - | - |
| Proportional Means Model - Supportive Analysis | | | |
| Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI | 2.00 (0.83, 4.86) | - | - |
| Total number of RBC transfusion in stratum 4 in RT phase | | | |
| N | 5 | 8 | 13 |
| Mean (SD) | 9.4 (6.35) | 4.3 (4.92) | 6.2 (5.86) |
| Median | 11.0 | 3.0 | 5.0 |
| Q1, Q3 | 7.0, 12.0 | 0.0, 7.5 | 0.0, 11.0 |
| Min, Max | 0.0, 17.0 | 0.0, 13.0 | 0.0, 17.0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

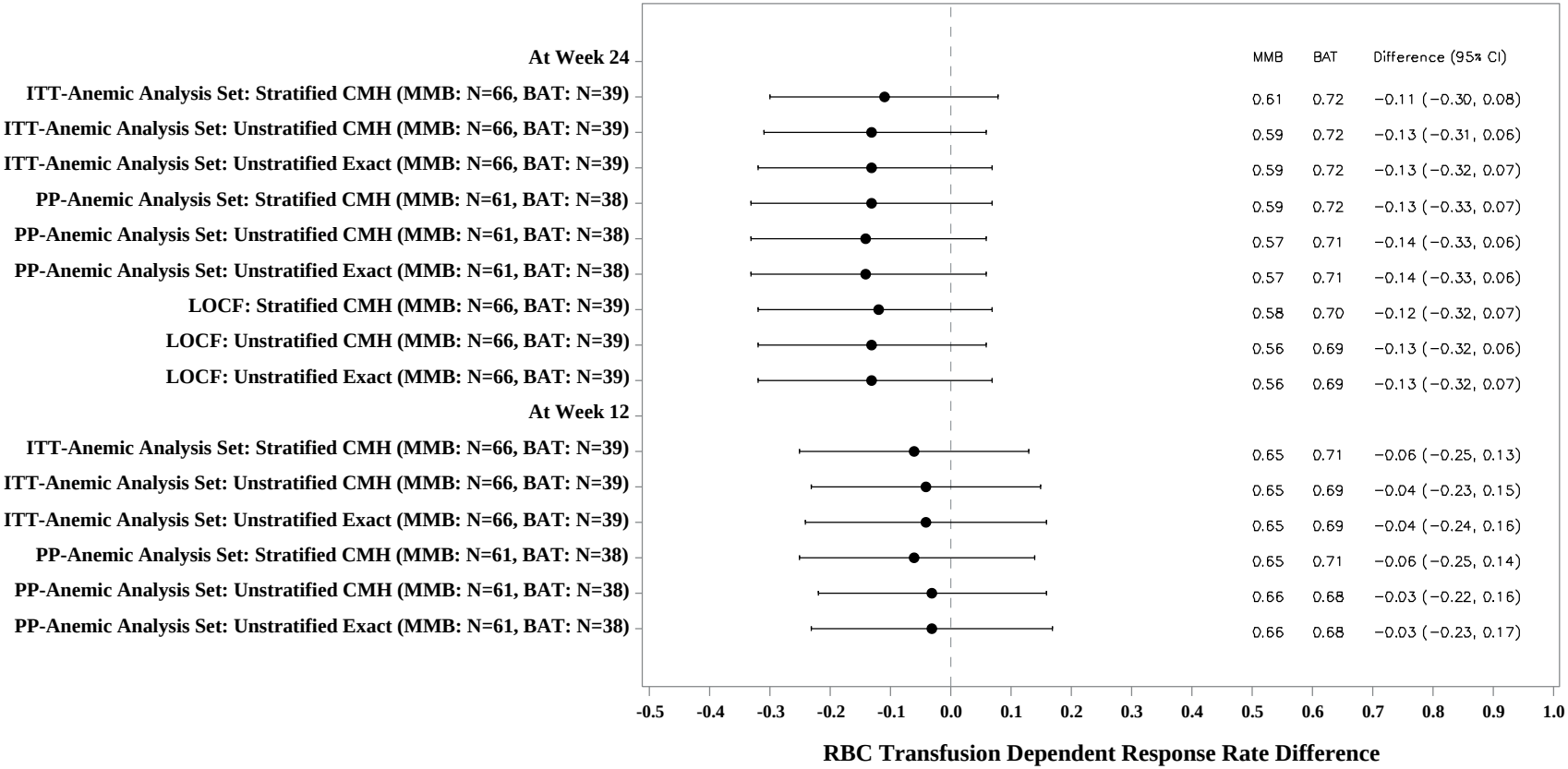
Table 2.2701: Sensitivity Analysis of Rate of RBC Transfusion
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Duration of the RT phase (months) in stratum 4 | | | |
| N | 5 | 8 | 13 |
| Mean (SD) | 5.26 (0.892) | 4.76 (1.529) | 4.95 (1.301) |
| Median | 5.55 | 5.55 | 5.55 |
| Q1, Q3 | 5.49, 5.78 | 4.01, 5.59 | 5.49, 5.62 |
| Min, Max | 3.68, 5.78 | 2.10, 5.65 | 2.10, 5.78 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
Proportional means model is Andersen-Gill model with robust sandwich variance estimate.
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Figure 2.2201: Forest Plot of Primary and Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set
All strata combined




ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
To the left of the reference line favors MMB, to the right favors BAT.
CMH = Cochran-Mantel-Haenszel; LOCF = Last Observation Carried Forward; PP=Per-Protocol; CI= Confidence Interval
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/g-rbc-td-forest.sas V.03.05 Output file: g-rbc-td-forest.pdf 25AUG2023:13:36

Table 2.3901: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **All Strata Combined** | | | |
| **RBC Transfusion Dependent Rate at Week 24** | | | |
| Not-Dependent, n(%) | 27 ( 40.9%) | 11 ( 28.2%) | 38 ( 36.2%) |
| Transfusion Requiring, n(%) | 5 ( 7.6%) | 6 ( 15.4%) | 11 ( 10.5%) |
| Transfusion Independent, n(%) | 22 ( 33.3%) | 5 ( 12.8%) | 27 ( 25.7%) |
| Dependent, n(%) | 39 ( 59.1%) | 28 ( 71.8%) | 67 ( 63.8%) |
| 95% Exact CI | 0.4629, 0.7105 | 0.5513, 0.8500 | 0.5385, 0.7296 |
| Proportion Difference - Stratified CMH Method (95% CI) | -0.11 (-0.30, 0.08) | | |
| p-value | 0.27 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | -0.13 (-0.31, 0.06) | | |
| p-value | 0.18 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | -0.13 (-0.32, 0.07) | | |
| p-value | 0.21 | | |
| Unadjusted Relative Risk (95% CI) | 0.82 (0.62, 1.09) | | |
| p-value [1] | 0.17 | | |
| Unadjusted Odds Ratio (95% CI) | 0.57 (0.24, 1.33) | | |
| p-value [1] | 0.19 | | |
| Unadjusted Absolute Risk Difference (95% CI) | -0.13 (-0.31, 0.06) | | |
| Adjusted Relative Risk (95% CI) [2] | 0.85 (0.64, 1.12) | | |
| p-value [2] | 0.24 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-tf-td-gba-rr.sas V.03.05 Output file: t-rbctd24-gba.pdf 24AUG2023:16:33

Table 2.3901: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| >=4 units transfused in the last 8 weeks | 14 ( 21.2%) | 15 ( 38.5%) | 29 ( 27.6%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 19 ( 28.8%) | 15 ( 38.5%) | 34 ( 32.4%) |
| Last Participation date < Day 162 in RT phase | 18 ( 27.3%) | 7 ( 17.9%) | 25 ( 23.8%) |
| Other | 7 ( 10.6%) | 8 ( 20.5%) | 15 ( 14.3%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3901: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **RBC Transfusion Dependent Rate at Week 12** | | | |
| Not-Dependent, n(%) | 23 ( 34.8%) | 12 ( 30.8%) | 35 ( 33.3%) |
| Transfusion Requiring, n(%) | 8 ( 12.1%) | 9 ( 23.1%) | 17 ( 16.2%) |
| Transfusion Independent, n(%) | 15 ( 22.7%) | 3 ( 7.7%) | 18 ( 17.1%) |
| Dependent, n(%) | 43 ( 65.2%) | 27 ( 69.2%) | 70 ( 66.7%) |
| 95% Exact CI | 0.5242, 0.7647 | 0.5243, 0.8298 | 0.5680, 0.7557 |
| Proportion Difference - Stratified CMH Method (95% CI) | -0.06 (-0.25, 0.13) | | |
| p-value | 0.54 | | |
| Proportion Difference - Unstratified CMH Method (95% CI) | -0.04 (-0.23, 0.15) | | |
| p-value | 0.67 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | -0.04 (-0.24, 0.16) | | |
| p-value | 0.83 | | |
| Unadjusted Relative Risk (95% CI) | 0.94 (0.72, 1.24) | | |
| p-value [1] | 0.66 | | |
| Unadjusted Odds Ratio (95% CI) | 0.83 (0.36, 1.94) | | |
| p-value [1] | 0.67 | | |
| Unadjusted Absolute Risk Difference (95% CI) | -0.04 (-0.23, 0.14) | | |
| Adjusted Relative Risk (95% CI) [2] | 0.92 (0.70, 1.19) | | |
| p-value [2] | 0.51 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3901: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| >=4 units transfused in the last 8 weeks | 26 ( 39.4%) | 13 ( 33.3%) | 39 ( 37.1%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 33 ( 50.0%) | 16 ( 41.0%) | 49 ( 46.7%) |
| Last Participation date < Day 78 in RT phase | 7 ( 10.6%) | 6 ( 15.4%) | 13 ( 12.4%) |
| Other | 13 ( 19.7%) | 9 ( 23.1%) | 22 ( 21.0%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3901: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Baseline TSS <18 | 35 | 14 | 49 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Not-Dependent, n(%) | 17 ( 48.6%) | 5 ( 35.7%) | 22 ( 44.9%) |
| Transfusion Requiring, n(%) | 3 ( 8.6%) | 5 ( 35.7%) | 8 ( 16.3%) |
| Transfusion Independent, n(%) | 14 ( 40.0%) | 0 | 14 ( 28.6%) |
| Dependent, n(%) | 18 ( 51.4%) | 9 ( 64.3%) | 27 ( 55.1%) |
| 95% Exact CI | 0.3399, 0.6862 | 0.3514, 0.8724 | 0.4023, 0.6933 |
| Proportion Difference (95% CI) | -0.13 (-0.44, 0.18) | | |
| Proportion Difference using Exact Method (95% CI) | -0.13 (-0.43, 0.19) | | |
| >=4 units transfused in the last 8 weeks | 6 ( 17.1%) | 5 ( 35.7%) | 11 ( 22.4%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 12 ( 34.3%) | 6 ( 42.9%) | 18 ( 36.7%) |
| Last Participation date < Day 162 in RT phase | 6 ( 17.1%) | 2 ( 14.3%) | 8 ( 16.3%) |
| Other | 6 ( 17.1%) | 5 ( 35.7%) | 11 ( 22.4%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3901: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **RBC Transfusion Dependent Rate at Week 12** | | | |
| Not-Dependent, n(%) | 13 ( 37.1%) | 4 ( 28.6%) | 17 ( 34.7%) |
| Transfusion Requiring, n(%) | 6 ( 17.1%) | 4 ( 28.6%) | 10 ( 20.4%) |
| Transfusion Independent, n(%) | 7 ( 20.0%) | 0 | 7 ( 14.3%) |
| Dependent, n(%) | 22 ( 62.9%) | 10 ( 71.4%) | 32 ( 65.3%) |
| 95% Exact CI | 0.4492, 0.7853 | 0.4190, 0.9161 | 0.5036, 0.7833 |
| Proportion Difference (95% CI) | -0.09 (-0.38, 0.21) | | |
| Proportion Difference using Exact Method (95% CI) | -0.09 (-0.39, 0.23) | | |
| >=4 units transfused in the last 8 weeks | 14 ( 40.0%) | 6 ( 42.9%) | 20 ( 40.8%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 21 ( 60.0%) | 6 ( 42.9%) | 27 ( 55.1%) |
| Last Participation date < Day 78 in RT phase | 1 ( 2.9%) | 2 ( 14.3%) | 3 ( 6.1%) |
| Other | 9 ( 25.7%) | 5 ( 35.7%) | 14 ( 28.6%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3901: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Baseline TSS >=18 | 17 | 11 | 28 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Not-Dependent, n(%) | 5 ( 29.4%) | 1 ( 9.1%) | 6 ( 21.4%) |
| Transfusion Requiring, n(%) | 2 ( 11.8%) | 0 | 2 ( 7.1%) |
| Transfusion Independent, n(%) | 3 ( 17.6%) | 1 ( 9.1%) | 4 ( 14.3%) |
| Dependent, n(%) | 12 ( 70.6%) | 10 ( 90.9%) | 22 ( 78.6%) |
| 95% Exact CI | 0.4404, 0.8969 | 0.5872, 0.9977 | 0.5905, 0.9170 |
| Proportion Difference (95% CI) | -0.20 (-0.51, 0.10) | | |
| Proportion Difference using Exact Method (95% CI) | -0.20 (-0.54, 0.17) | | |
| >=4 units transfused in the last 8 weeks | 5 ( 29.4%) | 7 ( 63.6%) | 12 ( 42.9%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 5 ( 29.4%) | 5 ( 45.5%) | 10 ( 35.7%) |
| Last Participation date < Day 162 in RT phase | 6 ( 35.3%) | 2 ( 18.2%) | 8 ( 28.6%) |
| Other | 1 ( 5.9%) | 2 ( 18.2%) | 3 ( 10.7%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3901: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **RBC Transfusion Dependent Rate at Week 12** | | | |
| Not-Dependent, n(%) | 3 ( 17.6%) | 1 ( 9.1%) | 4 ( 14.3%) |
| Transfusion Requiring, n(%) | 0 | 1 ( 9.1%) | 1 ( 3.6%) |
| Transfusion Independent, n(%) | 3 ( 17.6%) | 0 | 3 ( 10.7%) |
| Dependent, n(%) | 14 ( 82.4%) | 10 ( 90.9%) | 24 ( 85.7%) |
| 95% Exact CI | 0.5657, 0.9620 | 0.5872, 0.9977 | 0.6733, 0.9597 |
| Proportion Difference (95% CI) | -0.09 (-0.37, 0.20) | | |
| Proportion Difference using Exact Method (95% CI) | -0.09 (-0.44, 0.28) | | |
| >=4 units transfused in the last 8 weeks | 8 ( 47.1%) | 5 ( 45.5%) | 13 ( 46.4%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 9 ( 52.9%) | 7 ( 63.6%) | 16 ( 57.1%) |
| Last Participation date < Day 78 in RT phase | 3 ( 17.6%) | 2 ( 18.2%) | 5 ( 17.9%) |
| Other | 4 ( 23.5%) | 3 ( 27.3%) | 7 ( 25.0%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3901: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence No and Baseline TSS <18 | 7 | 6 | 13 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Not-Dependent, n(%) | 4 ( 57.1%) | 2 ( 33.3%) | 6 ( 46.2%) |
| Transfusion Requiring, n(%) | 0 | 0 | 0 |
| Transfusion Independent, n(%) | 4 ( 57.1%) | 2 ( 33.3%) | 6 ( 46.2%) |
| Dependent, n(%) | 3 ( 42.9%) | 4 ( 66.7%) | 7 ( 53.8%) |
| 95% Exact CI | 0.0990, 0.8159 | 0.2228, 0.9567 | 0.2513, 0.8078 |
| Proportion Difference (95% CI) | -0.24 (-0.82, 0.34) | | |
| Proportion Difference using Exact Method (95% CI) | -0.24 (-0.71, 0.34) | | |
| >=4 units transfused in the last 8 weeks | 0 | 1 ( 16.7%) | 1 ( 7.7%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 0 | 2 ( 33.3%) | 2 ( 15.4%) |
| Last Participation date < Day 162 in RT phase | 3 ( 42.9%) | 1 ( 16.7%) | 4 ( 30.8%) |
| Other | 0 | 1 ( 16.7%) | 1 ( 7.7%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3901: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **RBC Transfusion Dependent Rate at Week 12** | | | |
| Not-Dependent, n(%) | 4 ( 57.1%) | 3 ( 50.0%) | 7 ( 53.8%) |
| Transfusion Requiring, n(%) | 1 ( 14.3%) | 2 ( 33.3%) | 3 ( 23.1%) |
| Transfusion Independent, n(%) | 3 ( 42.9%) | 1 ( 16.7%) | 4 ( 30.8%) |
| Dependent, n(%) | 3 ( 42.9%) | 3 ( 50.0%) | 6 ( 46.2%) |
| 95% Exact CI | 0.0990, 0.8159 | 0.1181, 0.8819 | 0.1922, 0.7487 |
| Proportion Difference (95% CI) | -0.07 (-0.66, 0.52) | | |
| Proportion Difference using Exact Method (95% CI) | -0.07 (-0.59, 0.48) | | |
| >=4 units transfused in the last 8 weeks | 1 ( 14.3%) | 0 | 1 ( 7.7%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 1 ( 14.3%) | 2 ( 33.3%) | 3 ( 23.1%) |
| Last Participation date < Day 78 in RT phase | 2 ( 28.6%) | 0 | 2 ( 15.4%) |
| Other | 0 | 1 ( 16.7%) | 1 ( 7.7%) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3901: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Baseline TSS >=18 | 7 | 8 | 15 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Not-Dependent, n(%) | 1 ( 14.3%) | 3 ( 37.5%) | 4 ( 26.7%) |
| Transfusion Requiring, n(%) | 0 | 1 ( 12.5%) | 1 ( 6.7%) |
| Transfusion Independent, n(%) | 1 ( 14.3%) | 2 ( 25.0%) | 3 ( 20.0%) |
| Dependent, n(%) | 6 ( 85.7%) | 5 ( 62.5%) | 11 ( 73.3%) |
| 95% Exact CI | 0.4213, 0.9964 | 0.2449, 0.9148 | 0.4490, 0.9221 |
| Proportion Difference (95% CI) | 0.23 (-0.25, 0.71) | | |
| Proportion Difference using Exact Method (95% CI) | 0.23 (-0.26, 0.68) | | |
| >=4 units transfused in the last 8 weeks | 3 ( 42.9%) | 2 ( 25.0%) | 5 ( 33.3%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 2 ( 28.6%) | 2 ( 25.0%) | 4 ( 26.7%) |
| Last Participation date < Day 162 in RT phase | 3 ( 42.9%) | 2 ( 25.0%) | 5 ( 33.3%) |
| Other | 0 | 0 | 0 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.3901: Analysis of RBC Transfusion Dependence Rate at Week 24 and Week 12
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **RBC Transfusion Dependent Rate at Week 12** | | | |
| Not-Dependent, n(%) | 3 ( 42.9%) | 4 ( 50.0%) | 7 ( 46.7%) |
| Transfusion Requiring, n(%) | 1 ( 14.3%) | 2 ( 25.0%) | 3 ( 20.0%) |
| Transfusion Independent, n(%) | 2 ( 28.6%) | 2 ( 25.0%) | 4 ( 26.7%) |
| Dependent, n(%) | 4 ( 57.1%) | 4 ( 50.0%) | 8 ( 53.3%) |
| 95% Exact CI | 0.1841, 0.9010 | 0.1570, 0.8430 | 0.2659, 0.7873 |
| Proportion Difference (95% CI) | 0.07 (-0.47, 0.61) | | |
| Proportion Difference using Exact Method (95% CI) | 0.07 (-0.44, 0.56) | | |
| >=4 units transfused in the last 8 weeks | 3 ( 42.9%) | 2 ( 25.0%) | 5 ( 33.3%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 2 ( 28.6%) | 1 ( 12.5%) | 3 ( 20.0%) |
| Last Participation date < Day 78 in RT phase | 1 ( 14.3%) | 2 ( 25.0%) | 3 ( 20.0%) |
| Other | 0 | 0 | 0 |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.
[1] p-values were obtained from a Z-test.
[2] Adjusted Relative Risk and corresponding p-value were obtained from a modified Poisson regression model with robust sandwich matrix estimators, including treatment, baseline TD (yes, no), and baseline TSS (<18, >=18) as covariates.

Data Extracted: CRF data: 25JUN2019

Table 2.4401: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| **All Strata Combined** | | | |
| **RBC Transfusion Dependent Rate at Week 24** | | | |
| Not-Dependent, n(%) | 29 ( 43.9%) | 12 ( 30.8%) | 41 ( 39.0%) |
| Transfusion Requiring, n(%) | 5 ( 7.6%) | 7 ( 17.9%) | 12 ( 11.4%) |
| Transfusion Independent, n(%) | 24 ( 36.4%) | 5 ( 12.8%) | 29 ( 27.6%) |
| Dependent, n(%) | 37 ( 56.1%) | 27 ( 69.2%) | 64 ( 61.0%) |
| 95% Exact CI | 0.4330, 0.6826 | 0.5243, 0.8298 | 0.5094, 0.7033 |
| Proportion Difference - Stratified CMH Method(95% CI) | -0.12(-0.32, 0.07) | | |
| P-value | 0.22 | | |
| Proportion Difference - Unstratified Method (95% CI) | -0.13(-0.32, 0.06) | | |
| P-value | 0.18 | | |
| Proportion Difference - Unstratified Exact Method(95% CI) | -0.13(-0.32, 0.07) | | |
| P-value | 0.22 | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.4401: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Baseline TSS <18 | 35 | 14 | 49 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Not-Dependent, n(%) | 18 ( 51.4%) | 5 ( 35.7%) | 23 ( 46.9%) |
| Transfusion Requiring, n(%) | 3 ( 8.6%) | 5 ( 35.7%) | 8 ( 16.3%) |
| Transfusion Independent, n(%) | 15 ( 42.9%) | 0 | 15 ( 30.6%) |
| Dependent, n(%) | 17 ( 48.6%) | 9 ( 64.3%) | 26 ( 53.1%) |
| 95% Exact CI | 0.3138, 0.6601 | 0.3514, 0.8724 | 0.3827, 0.6747 |
| Proportion Difference (95% CI) | -0.16(-0.47, 0.15) | | |
| Proportion Difference using Exact Method(95% CI) | -0.16(-0.46, 0.16) | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.4401: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Baseline TSS >=18 | 17 | 11 | 28 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Not-Dependent, n(%) | 5 ( 29.4%) | 1 ( 9.1%) | 6 ( 21.4%) |
| Transfusion Requiring, n(%) | 2 ( 11.8%) | 0 | 2 ( 7.1%) |
| Transfusion Independent, n(%) | 3 ( 17.6%) | 1 ( 9.1%) | 4 ( 14.3%) |
| Dependent, n(%) | 12 ( 70.6%) | 10 ( 90.9%) | 22 ( 78.6%) |
| 95% Exact CI | 0.4404, 0.8969 | 0.5872, 0.9977 | 0.5905, 0.9170 |
| Proportion Difference (95% CI) | -0.20(-0.51, 0.10) | | |
| Proportion Difference using Exact Method(95% CI) | -0.20(-0.54, 0.17) | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.4401: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence No and Baseline TSS <18 | 7 | 6 | 13 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Not-Dependent, n(%) | 4 ( 57.1%) | 2 ( 33.3%) | 6 ( 46.2%) |
| Transfusion Requiring, n(%) | 0 | 0 | 0 |
| Transfusion Independent, n(%) | 4 ( 57.1%) | 2 ( 33.3%) | 6 ( 46.2%) |
| Dependent, n(%) | 3 ( 42.9%) | 4 ( 66.7%) | 7 ( 53.8%) |
| 95% Exact CI | 0.0990, 0.8159 | 0.2228, 0.9567 | 0.2513, 0.8078 |
| Proportion Difference (95% CI) | -0.24(-0.82, 0.34) | | |
| Proportion Difference using Exact Method(95% CI) | -0.24(-0.71, 0.34) | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.4401: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 -- LOCF
Randomized Treatment Phase
ITT-Anemic Analysis Set

| | MMB (N=66) | BAT (N=39) | Total (N=105) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Baseline TSS >=18 | 7 | 8 | 15 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Not-Dependent, n(%) | 2 ( 28.6%) | 4 ( 50.0%) | 6 ( 40.0%) |
| Transfusion Requiring, n(%) | 0 | 2 ( 25.0%) | 2 ( 13.3%) |
| Transfusion Independent, n(%) | 2 ( 28.6%) | 2 ( 25.0%) | 4 ( 26.7%) |
| Dependent, n(%) | 5 ( 71.4%) | 4 ( 50.0%) | 9 ( 60.0%) |
| 95% Exact CI | 0.2904, 0.9633 | 0.1570, 0.8430 | 0.3229, 0.8366 |
| Proportion Difference (95% CI) | 0.21(-0.31, 0.74) | | |
| Proportion Difference using Exact Method(95% CI) | 0.21(-0.31, 0.67) | | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent
LOCF = Last Observation Carried Forward
CMH = Cochran-Mantel-Haenszel;
RBC = Red Blood Cell

Data Extracted: CRF data: 25JUN2019

Table 2.4402: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **All Strata Combined** | | | |
| **RBC Transfusion Dependent Rate at Week 24** | | | |
| Not-Dependent, n(%) | 26 ( 42.6%) | 11 ( 28.9%) | 37 ( 37.4%) |
| Transfusion Requiring, n(%) | 5 ( 8.2%) | 6 ( 15.8%) | 11 ( 11.1%) |
| Transfusion Independent, n(%) | 21 ( 34.4%) | 5 ( 13.2%) | 26 ( 26.3%) |
| Dependent, n(%) | 35 ( 57.4%) | 27 ( 71.1%) | 62 ( 62.6%) |
| 95% Exact CI | 0.4406, 0.6996 | 0.5410, 0.8458 | 0.5233, 0.7215 |
| Proportion Difference - Stratified CMH Method (95% CI) | -0.13 (-0.33, 0.07) | | |
| p-value | 0.21 | | |
| Proportion Difference - Unstratified Method (95% CI) | -0.14 (-0.33, 0.06) | | |
| p-value | 0.16 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | -0.14 (-0.33, 0.06) | | |
| p-value | 0.20 | | |
| >=4 units transfused in the last 8 weeks | 13 ( 21.3%) | 14 ( 36.8%) | 27 ( 27.3%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 17 ( 27.9%) | 14 ( 36.8%) | 31 ( 31.3%) |
| Last Participation date < Day 162 in RT phase | 16 ( 26.2%) | 7 ( 18.4%) | 23 ( 23.2%) |
| Other | 6 ( 9.8%) | 7 ( 18.4%) | 13 ( 13.1%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 25JUN2019

Table 2.4402: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **RBC Transfusion Dependent Rate at Week 12** | | | |
| Not-Dependent, n(%) | 21 ( 34.4%) | 12 ( 31.6%) | 33 ( 33.3%) |
| Transfusion Requiring, n(%) | 8 ( 13.1%) | 9 ( 23.7%) | 17 ( 17.2%) |
| Transfusion Independent, n(%) | 13 ( 21.3%) | 3 ( 7.9%) | 16 ( 16.2%) |
| Dependent, n(%) | 40 ( 65.6%) | 26 ( 68.4%) | 66 ( 66.7%) |
| 95% Exact CI | 0.5231, 0.7727 | 0.5135, 0.8250 | 0.5648, 0.7582 |
| Proportion Difference - Stratified CMH Method (95% CI) | -0.06 (-0.25, 0.14) | | |
| p-value | 0.57 | | |
| Proportion Difference - Unstratified Method (95% CI) | -0.03 (-0.22, 0.16) | | |
| p-value | 0.77 | | |
| Proportion Difference - Unstratified Exact Method (95% CI) | -0.03 (-0.23, 0.17) | | |
| p-value | 0.83 | | |
| >=4 units transfused in the last 8 weeks | 24 ( 39.3%) | 13 ( 34.2%) | 37 ( 37.4%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 31 ( 50.8%) | 16 ( 42.1%) | 47 ( 47.5%) |
| Last Participation date < Day 78 in RT phase | 6 ( 9.8%) | 6 ( 15.8%) | 12 ( 12.1%) |
| Other | 11 ( 18.0%) | 8 ( 21.1%) | 19 ( 19.2%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-tf-td-sen.sas V.03.05 Output file: t-sen-ppt-rbctd24.pdf 24AUG2023:16:39

Table 2.4402: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Stratum 1 | | | |
| Transfusion Dependence Yes and Baseline TSS <18 | 32 | 14 | 46 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Not-Dependent, n(%) | 16 ( 50.0%) | 5 ( 35.7%) | 21 ( 45.7%) |
| Transfusion Requiring, n(%) | 3 ( 9.4%) | 5 ( 35.7%) | 8 ( 17.4%) |
| Transfusion Independent, n(%) | 13 ( 40.6%) | 0 | 13 ( 28.3%) |
| Dependent, n(%) | 16 ( 50.0%) | 9 ( 64.3%) | 25 ( 54.3%) |
| 95% Exact CI | 0.3189, 0.6811 | 0.3514, 0.8724 | 0.3901, 0.6910 |
| Proportion Difference (95% CI) | -0.14 (-0.46, 0.17) | | |
| Proportion Difference using Exact Method (95% CI) | -0.14 (-0.44, 0.18) | | |
| >=4 units transfused in the last 8 weeks | 5 ( 15.6%) | 5 ( 35.7%) | 10 ( 21.7%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 10 ( 31.3%) | 6 ( 42.9%) | 16 ( 34.8%) |
| Last Participation date < Day 162 in RT phase | 6 ( 18.8%) | 2 ( 14.3%) | 8 ( 17.4%) |
| Other | 5 ( 15.6%) | 5 ( 35.7%) | 10 ( 21.7%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 25JUN2019

Table 2.4402: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **RBC Transfusion Dependent Rate at Week 12** | | | |
| Not-Dependent, n(%) | 12 ( 37.5%) | 4 ( 28.6%) | 16 ( 34.8%) |
| Transfusion Requiring, n(%) | 6 ( 18.8%) | 4 ( 28.6%) | 10 ( 21.7%) |
| Transfusion Independent, n(%) | 6 ( 18.8%) | 0 | 6 ( 13.0%) |
| Dependent, n(%) | 20 ( 62.5%) | 10 ( 71.4%) | 30 ( 65.2%) |
| 95% Exact CI | 0.4369, 0.7890 | 0.4190, 0.9161 | 0.4975, 0.7865 |
| Proportion Difference (95% CI) | -0.09(-0.39, 0.21) | | |
| Proportion Difference using Exact Method (95% CI) | -0.09 (-0.39, 0.22) | | |
| >=4 units transfused in the last 8 weeks | 12 ( 37.5%) | 6 ( 42.9%) | 18 ( 39.1%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 19 ( 59.4%) | 6 ( 42.9%) | 25 ( 54.3%) |
| Last Participation date < Day 78 in RT phase | 1 ( 3.1%) | 2 ( 14.3%) | 3 ( 6.5%) |
| Other | 7 ( 21.9%) | 5 ( 35.7%) | 12 ( 26.1%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-tf-td-sen.sas V.03.05 Output file: t-sen-ppt-rbctd24.pdf 24AUG2023:16:39

Table 2.4402: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Stratum 2 | | | |
| Transfusion Dependence Yes and Baseline TSS >=18 | 17 | 10 | 27 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Not-Dependent, n(%) | 5 ( 29.4%) | 1 ( 10.0%) | 6 ( 22.2%) |
| Transfusion Requiring, n(%) | 2 ( 11.8%) | 0 | 2 ( 7.4%) |
| Transfusion Independent, n(%) | 3 ( 17.6%) | 1 ( 10.0%) | 4 ( 14.8%) |
| Dependent, n(%) | 12 ( 70.6%) | 9 ( 90.0%) | 21 ( 77.8%) |
| 95% Exact CI | 0.4404, 0.8969 | 0.5550, 0.9975 | 0.5774, 0.9138 |
| Proportion Difference (95% CI) | -0.19 (-0.51, 0.12) | | |
| Proportion Difference using Exact Method (95% CI) | -0.19 (-0.54, 0.19) | | |
| >=4 units transfused in the last 8 weeks | 5 ( 29.4%) | 6 ( 60.0%) | 11 ( 40.7%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 5 ( 29.4%) | 4 ( 40.0%) | 9 ( 33.3%) |
| Last Participation date < Day 162 in RT phase | 6 ( 35.3%) | 2 ( 20.0%) | 8 ( 29.6%) |
| Other | 1 ( 5.9%) | 1 ( 10.0%) | 2 ( 7.4%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 25JUN2019

Table 2.4402: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **RBC Transfusion Dependent Rate at Week 12** | | | |
| Not-Dependent, n(%) | 3 ( 17.6%) | 1 ( 10.0%) | 4 ( 14.8%) |
| Transfusion Requiring, n(%) | 0 | 1 ( 10.0%) | 1 ( 3.7%) |
| Transfusion Independent, n(%) | 3 ( 17.6%) | 0 | 3 ( 11.1%) |
| Dependent, n(%) | 14 ( 82.4%) | 9 ( 90.0%) | 23 ( 85.2%) |
| 95% Exact CI | 0.5657, 0.9620 | 0.5550, 0.9975 | 0.6627, 0.9581 |
| Proportion Difference (95% CI) | -0.08(-0.37, 0.22) | | |
| Proportion Difference using Exact Method (95% CI) | -0.08 (-0.44, 0.30) | | |
| >=4 units transfused in the last 8 weeks | 8 ( 47.1%) | 5 ( 50.0%) | 13 ( 48.1%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 9 ( 52.9%) | 7 ( 70.0%) | 16 ( 59.3%) |
| Last Participation date < Day 78 in RT phase | 3 ( 17.6%) | 2 ( 20.0%) | 5 ( 18.5%) |
| Other | 4 ( 23.5%) | 2 ( 20.0%) | 6 ( 22.2%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-tf-td-sen.sas V.03.05 Output file: t-sen-ppt-rbctd24.pdf 24AUG2023:16:39

Table 2.4402: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Stratum 3 | | | |
| Transfusion Dependence No and Baseline TSS <18 | 7 | 6 | 13 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Not-Dependent, n(%) | 4 ( 57.1%) | 2 ( 33.3%) | 6 ( 46.2%) |
| Transfusion Requiring, n(%) | 0 | 0 | 0 |
| Transfusion Independent, n(%) | 4 ( 57.1%) | 2 ( 33.3%) | 6 ( 46.2%) |
| Dependent, n(%) | 3 ( 42.9%) | 4 ( 66.7%) | 7 ( 53.8%) |
| 95% Exact CI | 0.0990, 0.8159 | 0.2228, 0.9567 | 0.2513, 0.8078 |
| Proportion Difference (95% CI) | -0.24 (-0.82, 0.34) | | |
| Proportion Difference using Exact Method (95% CI) | -0.24 (-0.71, 0.34) | | |
| >=4 units transfused in the last 8 weeks | 0 | 1 ( 16.7%) | 1 ( 7.7%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 0 | 2 ( 33.3%) | 2 ( 15.4%) |
| Last Participation date < Day 162 in RT phase | 3 ( 42.9%) | 1 ( 16.7%) | 4 ( 30.8%) |
| Other | 0 | 1 ( 16.7%) | 1 ( 7.7%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 25JUN2019

Table 2.4402: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **RBC Transfusion Dependent Rate at Week 12** | | | |
| Not-Dependent, n(%) | 4 ( 57.1%) | 3 ( 50.0%) | 7 ( 53.8%) |
| Transfusion Requiring, n(%) | 1 ( 14.3%) | 2 ( 33.3%) | 3 ( 23.1%) |
| Transfusion Independent, n(%) | 3 ( 42.9%) | 1 ( 16.7%) | 4 ( 30.8%) |
| Dependent, n(%) | 3 ( 42.9%) | 3 ( 50.0%) | 6 ( 46.2%) |
| 95% Exact CI | 0.0990, 0.8159 | 0.1181, 0.8819 | 0.1922, 0.7487 |
| Proportion Difference (95% CI) | -0.07(-0.66, 0.52) | | |
| Proportion Difference using Exact Method (95% CI) | -0.07 (-0.59, 0.48) | | |
| >=4 units transfused in the last 8 weeks | 1 ( 14.3%) | 0 | 1 ( 7.7%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 1 ( 14.3%) | 2 ( 33.3%) | 3 ( 23.1%) |
| Last Participation date < Day 78 in RT phase | 2 ( 28.6%) | 0 | 2 ( 15.4%) |
| Other | 0 | 1 ( 16.7%) | 1 ( 7.7%) |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 25JUN2019

Table 2.4402: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| Stratum 4 | | | |
| Transfusion Dependence No and Baseline TSS >=18 | 5 | 8 | 13 |
| RBC Transfusion Dependent Rate at Week 24 | | | |
| Not-Dependent, n(%) | 1 ( 20.0%) | 3 ( 37.5%) | 4 ( 30.8%) |
| Transfusion Requiring, n(%) | 0 | 1 ( 12.5%) | 1 ( 7.7%) |
| Transfusion Independent, n(%) | 1 ( 20.0%) | 2 ( 25.0%) | 3 ( 23.1%) |
| Dependent, n(%) | 4 ( 80.0%) | 5 ( 62.5%) | 9 ( 69.2%) |
| 95% Exact CI | 0.2836, 0.9949 | 0.2449, 0.9148 | 0.3857, 0.9091 |
| Proportion Difference (95% CI) | 0.18 (-0.38, 0.73) | | |
| Proportion Difference using Exact Method (95% CI) | 0.18 (-0.40, 0.66) | | |
| >=4 units transfused in the last 8 weeks | 3 ( 60.0%) | 2 ( 25.0%) | 5 ( 38.5%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 2 ( 40.0%) | 2 ( 25.0%) | 4 ( 30.8%) |
| Last Participation date < Day 162 in RT phase | 1 ( 20.0%) | 2 ( 25.0%) | 3 ( 23.1%) |
| Other | 0 | 0 | 0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 25JUN2019

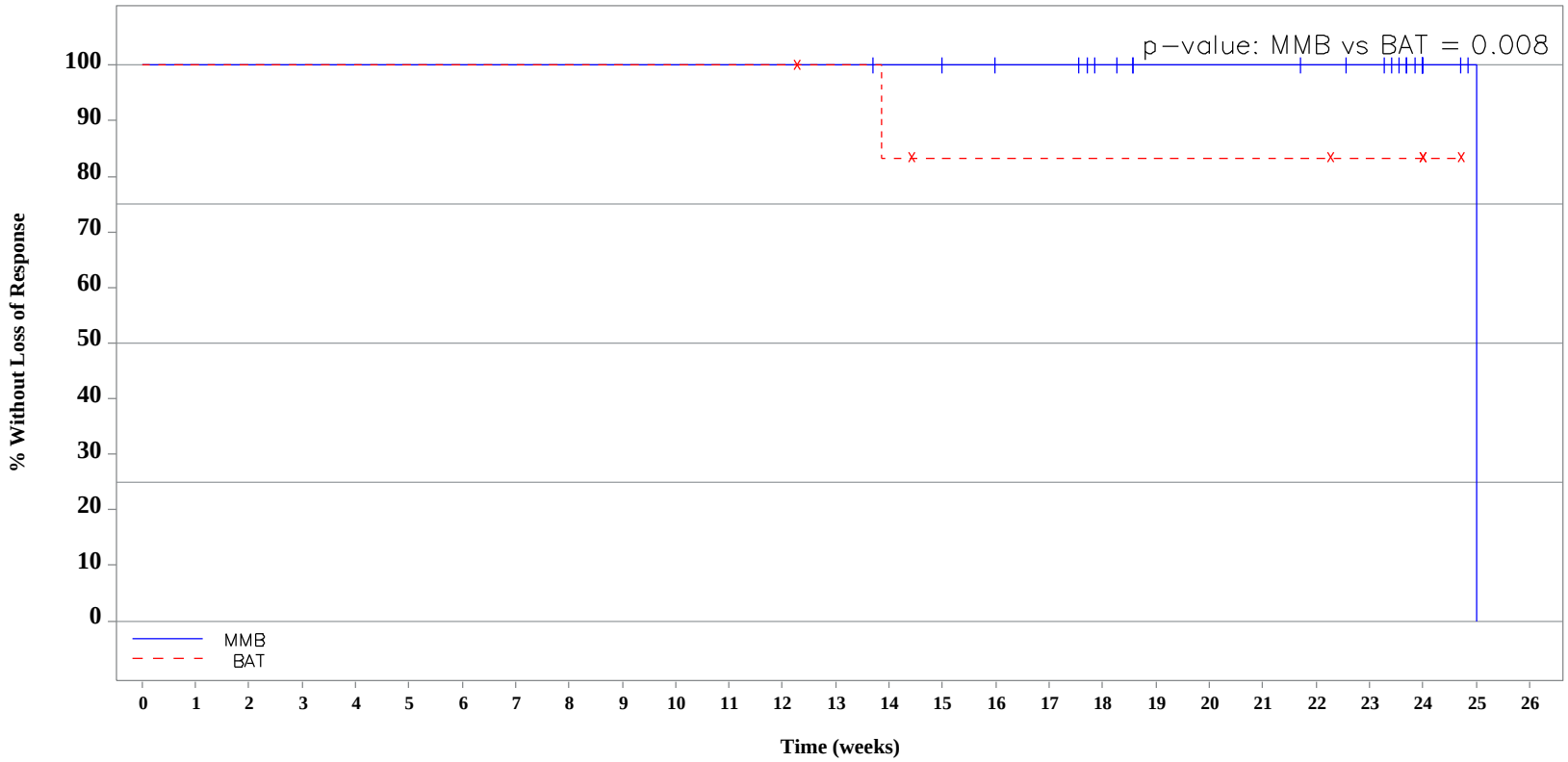
Table 2.4402: Sensitivity Analysis of RBC Transfusion Dependent Response Rate at Week 24 and at Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set

| | MMB (N=61) | BAT (N=38) | Total (N=99) |
|---|---|---|---|
| **RBC Transfusion Dependent Rate at Week 12** | | | |
| Not-Dependent, n(%) | 2 ( 40.0%) | 4 ( 50.0%) | 6 ( 46.2%) |
| Transfusion Requiring, n(%) | 1 ( 20.0%) | 2 ( 25.0%) | 3 ( 23.1%) |
| Transfusion Independent, n(%) | 1 ( 20.0%) | 2 ( 25.0%) | 3 ( 23.1%) |
| Dependent, n(%) | 3 ( 60.0%) | 4 ( 50.0%) | 7 ( 53.8%) |
| 95% Exact CI | 0.1466, 0.9473 | 0.1570, 0.8430 | 0.2513, 0.8078 |
| Proportion Difference (95% CI) | 0.10(-0.51, 0.71) | | |
| Proportion Difference using Exact Method (95% CI) | 0.10 (-0.46, 0.61) | | |
| >=4 units transfused in the last 8 weeks | 3 ( 60.0%) | 2 ( 25.0%) | 5 ( 38.5%) |
| Any Hgb assessment < 8g/dL in the last 8 weeks | 2 ( 40.0%) | 1 ( 12.5%) | 3 ( 23.1%) |
| Last Participation date < Day 78 in RT phase | 0 | 2 ( 25.0%) | 2 ( 15.4%) |
| Other | 0 | 0 | 0 |

PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel.
RBC = Red Blood Cell.

Data Extracted: CRF data: 25JUN2019

Figure 2.2201: Kaplan-Meier Plot of Duration of RBC Transfusion Independent Response
Randomized Treatment Phase
ITT-Anemic Analysis Set With RBC Transfusion Independent Response




**N at Risk (Events)**

| | | | | | | | | | | | | | | | | | | | | | | | | | | |
|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|
| **MMB** | 25 (0) | 25 (0) | 25 (0) | 25 (0) | 25 (0) | 25 (0) | 25 (0) | 25 (0) | 25 (0) | 25 (0) | 25 (0) | 25 (0) | 25 (0) | 25 (0) | 24 (0) | 24 (0) | 23 (0) | 22 (0) | 19 (0) | 16 (0) | 16 (0) | 16 (0) | 15 (0) | 14 (0) | 8 (0) | 1 (1) | 0 (1) |
| **BAT** | 7 (0) | 7 (0) | 7 (0) | 7 (0) | 7 (0) | 7 (0) | 7 (0) | 7 (0) | 7 (0) | 7 (0) | 7 (0) | 7 (0) | 7 (0) | 6 (0) | 5 (1) | 4 (1) | 4 (1) | 4 (1) | 4 (1) | 4 (1) | 4 (1) | 4 (1) | 4 (1) | 3 (1) | 3 (1) | 0 (1) | 0 (1) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
Stratified two-sided p-value is from a log-rank test comparing treatment stratified by baseline TD (yes,no) and baseline TSS (<18,>=18).

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/g-dur-rbc-ti_Sep2023.sas V.03.05 Output file: g-dur-rbc-ti.pdf 27SEP2023:16:21

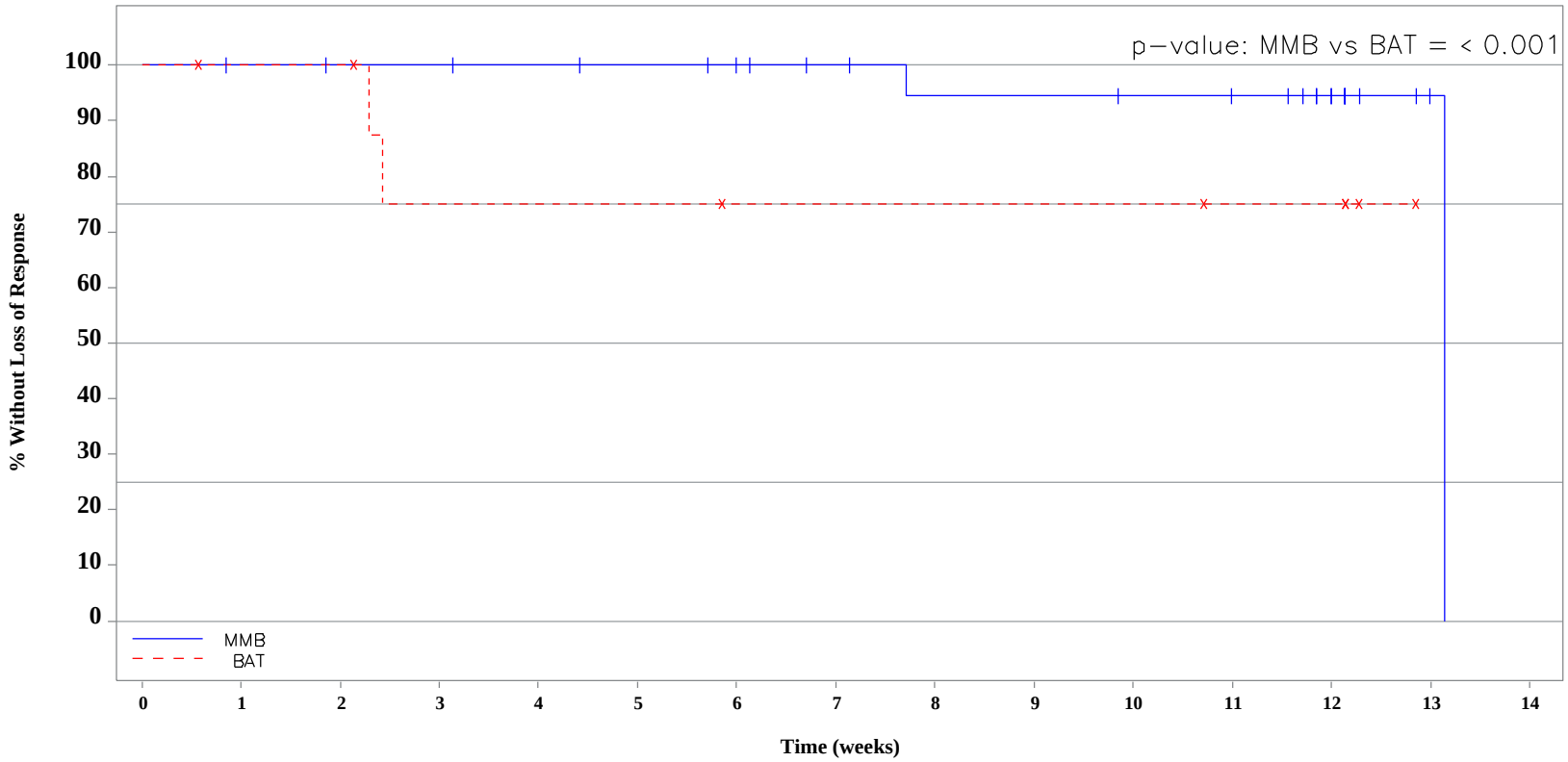
Table 2.8601: Analysis of Duration of RBC Transfusion Independent Response
Randomized Treatment Phase
ITT-Anemic Analysis Set with RBC Transfusion Independent Response

| | MMB (N=25) | BAT (N=7) |
|---|---|---|
| Subjects with Event | | |
| Loss of RBC Transfusion Independent Response, n(%) | 1 ( 4.0%) | 1 ( 14.3%) |
| Censor | | |
| Subjects Censored, n(%) | 24 ( 96.0%) | 6 ( 85.7%) |
| No Loss of Response: Censored at Last Subject Visit Date at RT Phase | 24 (100.0%) | 6 (100.0%) |
| Kaplan-Meier Estimate of Duration of RBC Transfusion Independent Response (Weeks) | | |
| 25-percentile (95% CI) | 25.00 (NE, NE) | NE (13.86, NE) |
| Median (95% CI) | 25.00 (NE, NE) | NE (13.86, NE) |
| 75-percentile (95% CI) | 25.00 (NE, NE) | NE (NE, NE) |
| Min, Max | 13.71, 25.00 | 12.29, 24.71 |
| Stratified Log-Rank Test p-value | 0.008 | |
| Adjusted Hazard Ratio (95% CI) | <0.01 (<0.01, NE) | |
| Unstratified Log-Rank Test p-value | 0.046 | |
| Unadjusted Hazard Ratio (95% CI) | <0.01 (<0.01, NE) | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
RBC transfusion independent response is defined as the absence of RBC transfusions and no hemoglobin level below 8 g/dL in the prior 12 weeks.
Stratified two-sided p-value is from a log-rank test comparing treatment stratified by baseline TD (yes,no) and baseline TSS (<18,>=18).
Adjusted hazard ratio and 95% CI are from a Cox proportional hazards model with covariates of treatment, baseline TD (yes,no) and baseline TSS (<18,>=18). Unstratified two-sided p-value is from a log-rank test comparing treatment.
Unadjusted hazard ratio and 95% CI are from a Cox proportional hazards model with a covariate of treatment.
NE = Not estimable.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-dur-rbc-ti_Sep2023.sas V.03.05 Output file: t-dur-rbc-ti.pdf 27SEP2023:16:24

Figure 2.2501: Kaplan-Meier Plot of Duration of Any Type Transfusion Free Response
Randomized Treatment Phase
ITT-Anemic Analysis Set With Any Type Transfusion Free Response



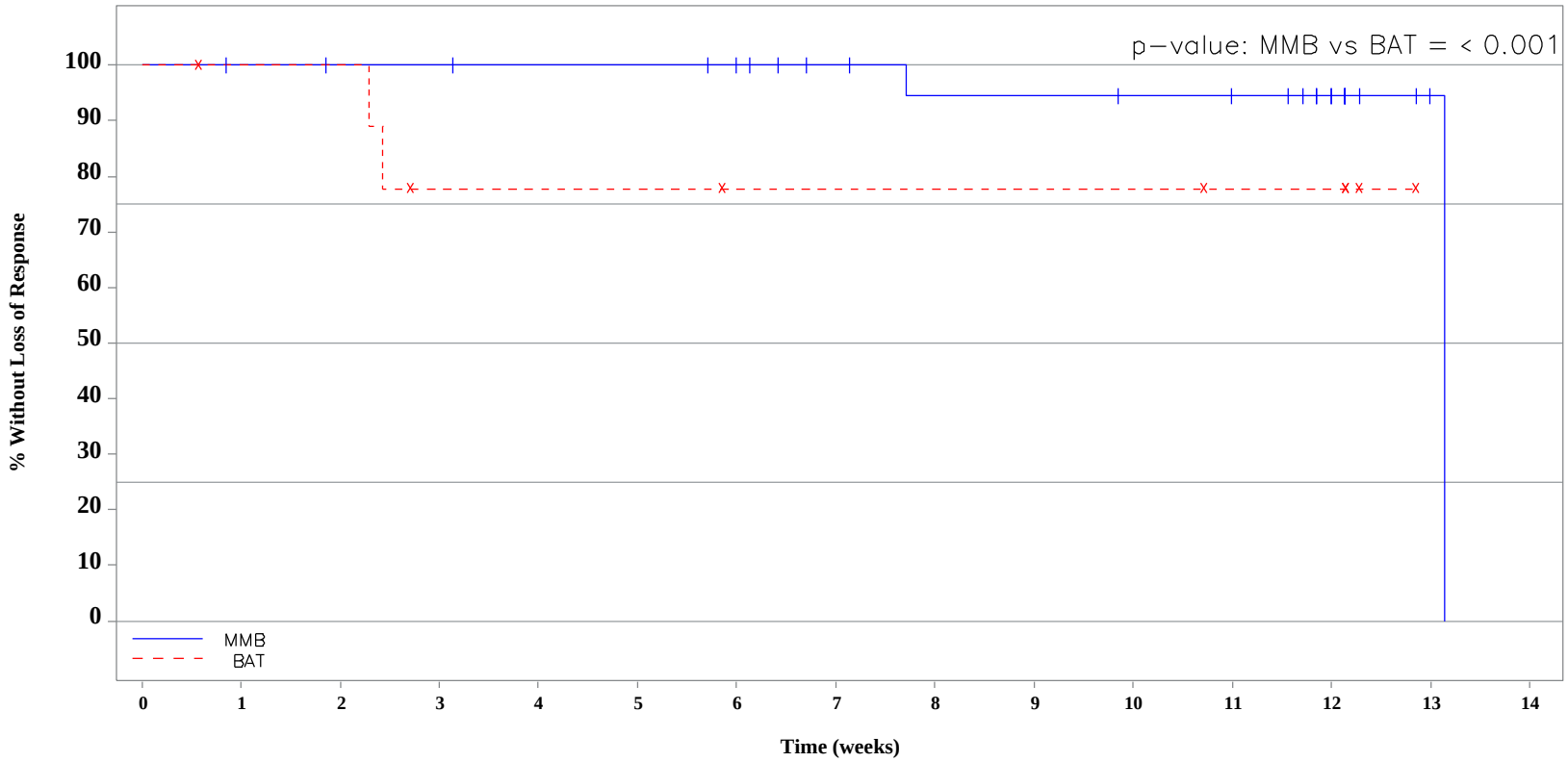

**N at Risk (Events)**

| | | | | | | | | | | | | | | |
|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|
| **MMB** | 27 (0) | 26 (0) | 25 (0) | 25 (0) | 24 (0) | 23 (0) | 22 (0) | 19 (0) | 17 (1) | 17 (1) | 16 (1) | 16 (1) | 11 (1) | 2 (1) | 0 (2) |
| **BAT** | 10 (0) | 9 (0) | 9 (0) | 6 (2) | 6 (2) | 6 (2) | 5 (2) | 5 (2) | 5 (2) | 5 (2) | 5 (2) | 4 (2) | 4 (2) | 0 (2) | 0 (2) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
Stratified two-sided p-value is from a log-rank test comparing treatment stratified by baseline TD (yes,no) and baseline TSS (<18,>=18).

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/g-dur-any-tfr_Sep2023.sas V.03.05 Output file: g-dur-any-tfr.pdf 27SEP2023:16:22

Figure 2.2301: Kaplan-Meier Plot of Duration of RBC Transfusion Free Response
Randomized Treatment Phase
ITT-Anemic Analysis Set With RBC Transfusion Free Response




**N at Risk (Events)**

| | | | | | | | | | | | | | | |
|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|
| **MMB** | 27 (0) | 26 (0) | 25 (0) | 25 (0) | 24 (0) | 24 (0) | 23 (0) | 19 (0) | 17 (1) | 17 (1) | 16 (1) | 16 (1) | 11 (1) | 2 (1) | 0 (2) |
| **BAT** | 10 (0) | 9 (0) | 9 (0) | 6 (2) | 6 (2) | 6 (2) | 5 (2) | 5 (2) | 5 (2) | 5 (2) | 5 (2) | 4 (2) | 4 (2) | 0 (2) | 0 (2) |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
Stratified two-sided p-value is from a log-rank test comparing treatment stratified by baseline TD (yes,no) and baseline TSS (<18,>=18).

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/g-dur-rbc-tfr_Sep2023.sas V.03.05 Output file: g-dur-rbc-tfr.pdf 27SEP2023:16:21

Table 2.5401: Analysis of Duration of Any Type Transfusion Free Response
Randomized Treatment Phase
ITT-Anemic Analysis Set Set With Any Type Transfusion Free Response

| | MMB (N=27) | BAT (N=10) |
|---|---|---|
| Subjects with Event | | |
| Loss of Any Type Transfusion Free Response, n(%) | 2 ( 7.4%) | 2 ( 20.0%) |
| Censor | | |
| Subjects Censored, n(%) | 25 ( 92.6%) | 8 ( 80.0%) |
| No Loss of Response: Censored at Last Subject Visit Date at RT Phase | 25 (100.0%) | 8 (100.0%) |
| Kaplan-Meier Estimate of Duration of Any Type Transfusion Free Response (Weeks) | | |
| 25-percentile (95% CI) | 13.14 (7.71, NE) | NE (2.29, NE) |
| Median (95% CI) | 13.14 (NE, NE) | NE (2.29, NE) |
| 75-percentile (95% CI) | 13.14 (NE, NE) | NE (NE, NE) |
| Min, Max | 0.86, 13.14 | 0.57, 12.86 |
| Stratified Log-Rank Test p-value | < 0.001 | |
| Adjusted Hazard Ratio (95% CI) | <0.01 (<0.01, NE) | |
| Unstratified Log-Rank Test p-value | 0.068 | |
| Unadjusted Hazard Ratio (95% CI) | 0.15 (0.01, 1.61) | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
Any type transfusion free response is defined as the absence of transfusion of any type in the prior 12 weeks.
Stratified two-sided p-value is from a log-rank test comparing treatment stratified by baseline TD (yes,no) and baseline TSS (<18,>=18).
Adjusted hazard ratio and 95% CI are from a Cox proportional hazards model with covariates of treatment, baseline TD (yes,no) and baseline TSS (<18,>=18). Unstratified two-sided p-value is from a log-rank test comparing treatment.
Unadjusted hazard ratio and 95% CI are from a Cox proportional hazards model with a covariate of treatment.
NE = Not estimable.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-dur-any-tfr_Sep2023.sas V.03.05 Output file: t-dur-any-tfr.pdf 26SEP2023:16:52

Table 2.4901: Analysis of Duration of RBC Transfusion Free Response
Randomized Treatment Phase
ITT-Anemic Analysis Set With RBC Transfusion Free Response

| | MMB (N=27) | BAT (N=10) |
|---|---|---|
| Subjects with Event | | |
| Loss of RBC Transfusion Free Response, n(%) | 2 ( 7.4%) | 2 ( 20.0%) |
| Censor | | |
| Subjects Censored, n(%) | 25 ( 92.6%) | 8 ( 80.0%) |
| No Loss of Response: Censored at Last Subject Visit Date at RT Phase | 25 (100.0%) | 8 (100.0%) |
| Kaplan-Meier Estimate of Duration of RBC Transfusion Free Response (Weeks) | | |
| 25-percentile (95% CI) | 13.14 (7.71, NE) | NE (2.29, NE) |
| Median (95% CI) | 13.14 (NE, NE) | NE (2.29, NE) |
| 75-percentile (95% CI) | 13.14 (NE, NE) | NE (NE, NE) |
| Min, Max | 0.86, 13.14 | 0.57, 12.86 |
| Stratified Log-Rank Test p-value | < 0.001 | |
| Adjusted Hazard Ratio (95% CI) | <0.01 (<0.01, NE) | |
| Unstratified Log-Rank Test p-value | 0.085 | |
| Unadjusted Hazard Ratio (95% CI) | 0.16 (0.01, 1.75) | |

ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
RBC transfusion free response is defined as the absence of RBC transfusions in the prior 12 weeks.
Stratified two-sided p-value is from a log-rank test comparing treatment stratified by baseline TD (yes,no) and baseline TSS (<18,>=18).
Adjusted hazard ratio and 95% CI are from a Cox proportional hazards model with covariates of treatment, baseline TD (yes,no) and baseline TSS (<18,>=18). Unstratified two-sided p-value is from a log-rank test comparing treatment.
Unadjusted hazard ratio and 95% CI are from a Cox proportional hazards model with a covariate of treatment.
NE = Not estimable.

Data Extracted: CRF data: 25JUN2019
Source: /mnt/code/SSAP/G-BA/prod/tfls/t-dur-rbc-tfr_Sep2023.sas V.03.05 Output file: t-dur-rbc-tfr.pdf 27SEP2023:16:24