Momelotinib (Omjjara)

GlaxoSmithKline GmbH & Co. KG

Anhang 4-H zu Modul 4A

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: 31.10.2025

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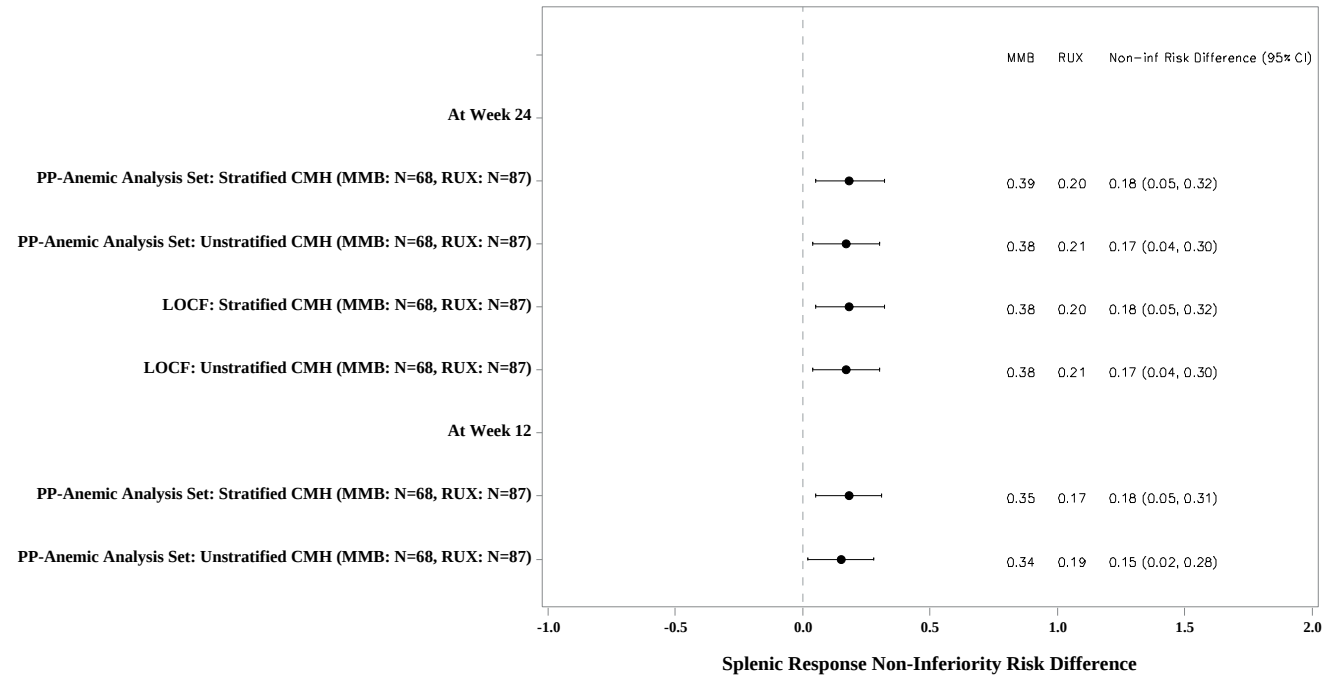
1 SIMPLIFY-1

1.1 Milzansprechen

1.1.1 Pre-defined

GSK Oncology
Study GS-US-352-0101

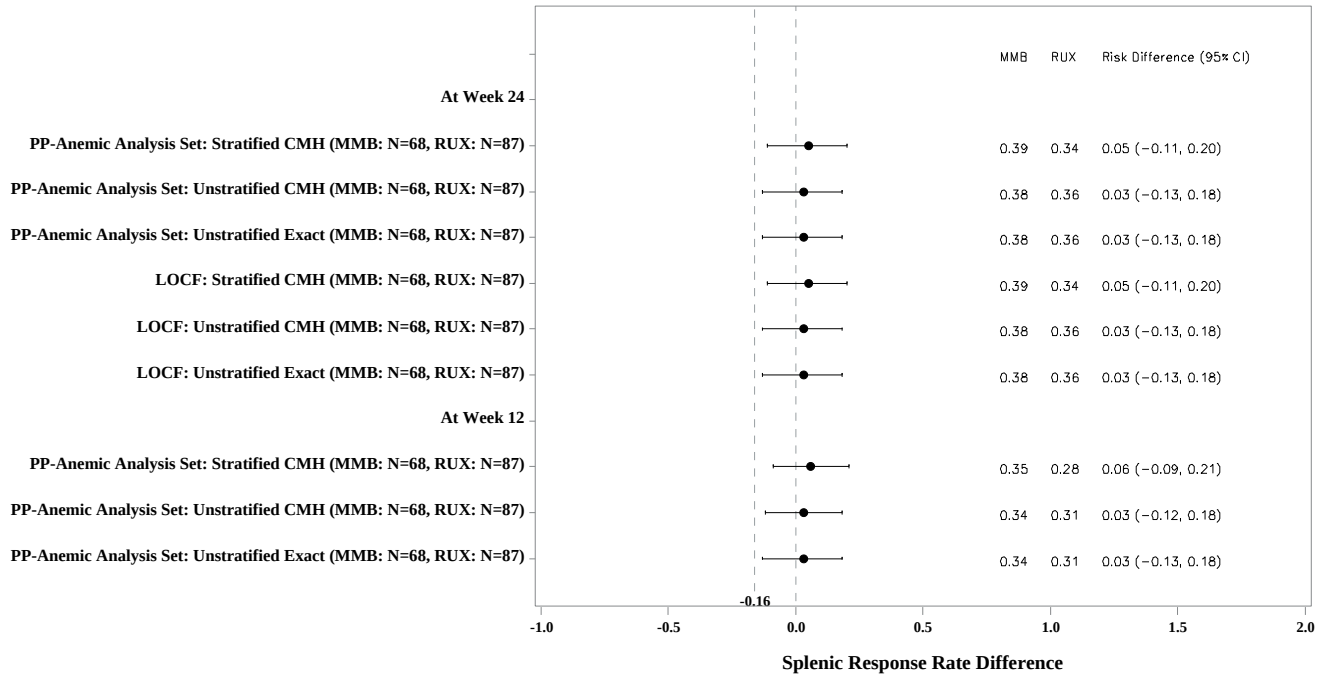
Figure 2.0202: Forest plot of Primary and Sensitivity Analysis of Splenic Response Rate at Week 24 and Week 12
Double-Blind Phase
PP-Anemic Analysis Set
Noninferiority



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



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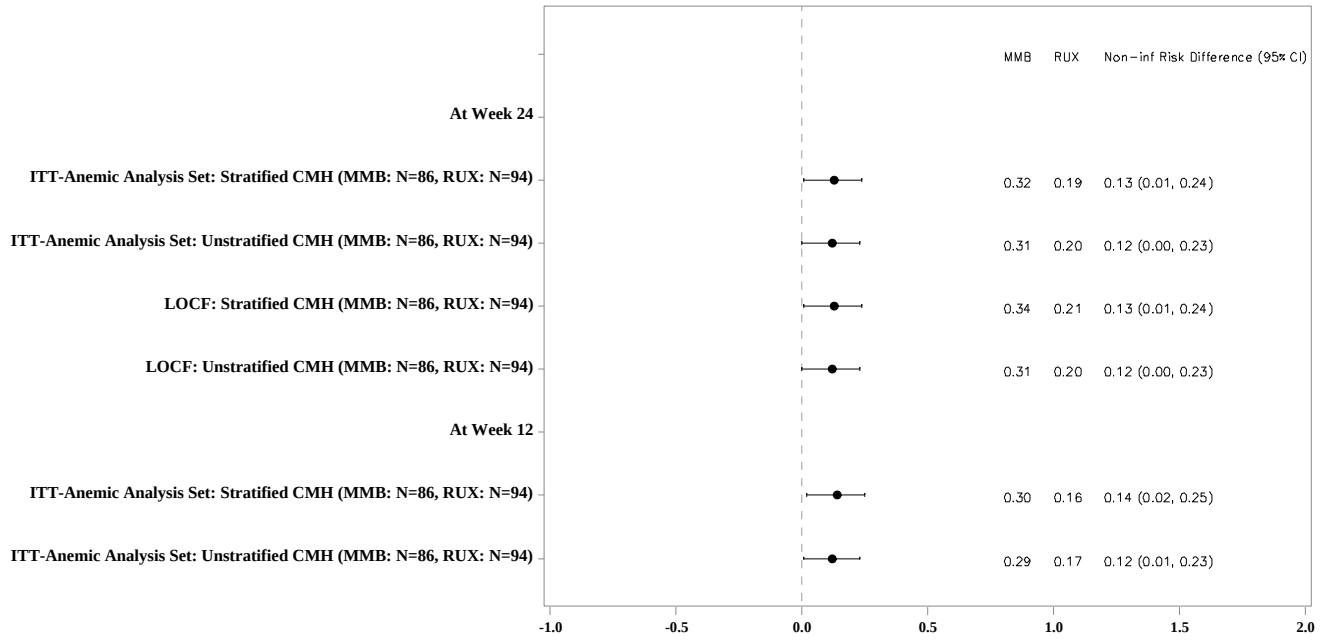
Data Extracted: CRF data: 01JUL2019
Source: g-srr-forest.sas V.03.05 Output file: g-srr-forest-pp.pdf 30AUG2023:12:27

Figure 2.0201: 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

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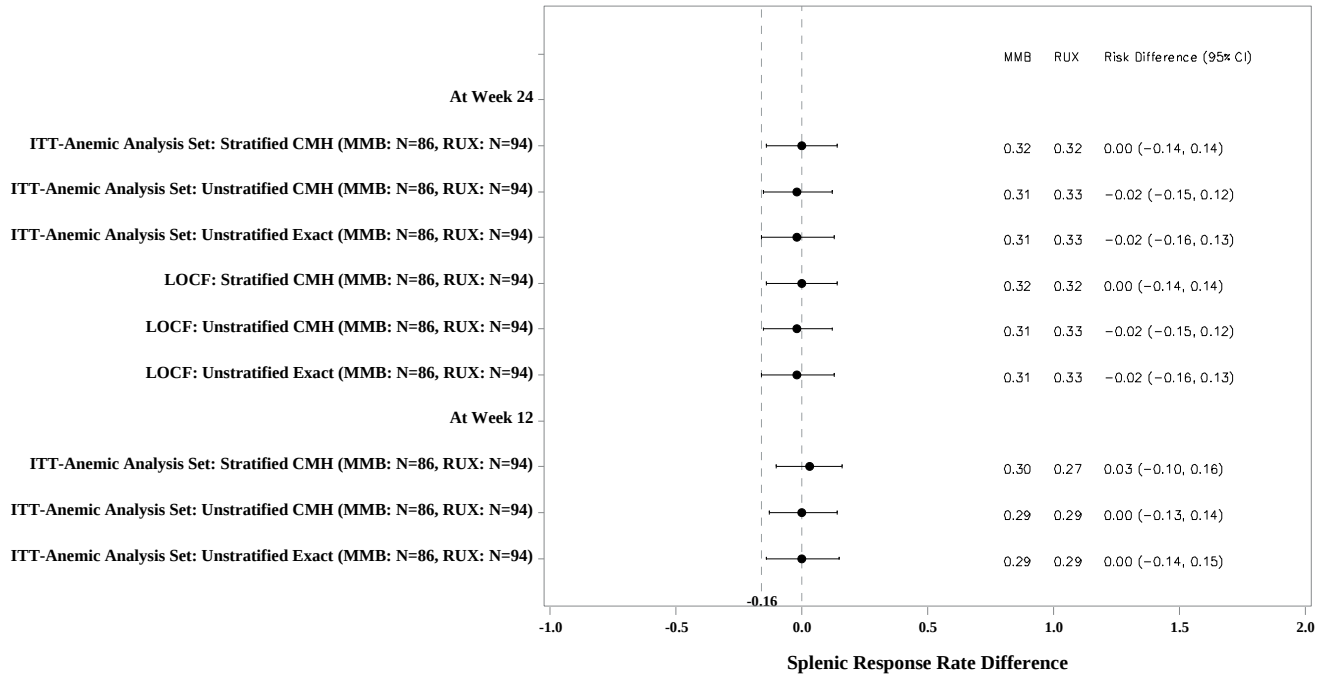
Source: g-srr-forest.sas V.03.05 Output file: g-srr-forest.pdf 30AUG2023:12:27

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



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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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.

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 (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.

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(%)	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.

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 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.

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(%)	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.

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 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

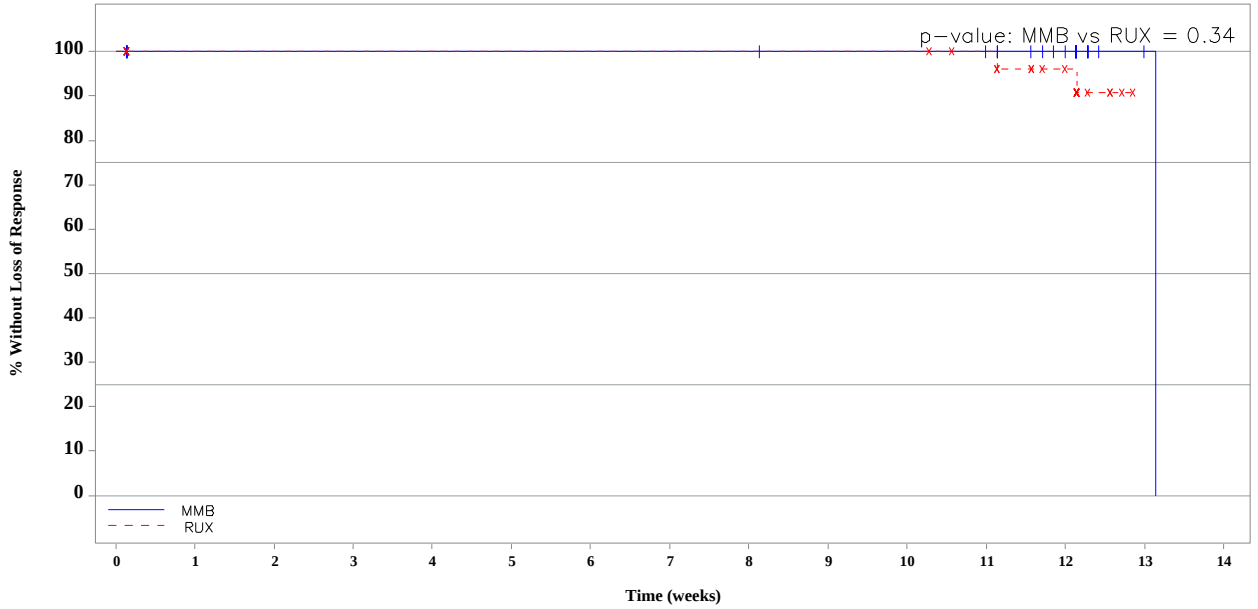
	MMB (N=86)	RUX (N=94)	Total (N=180)
Splenic Response Rate at Week 24			
Responder, n(%)	6 (33.3%)	15 (48.4%)	21 (42.9%)
95% Exact CI	0.1334, 0.5901	0.3015, 0.6694	0.2882, 0.5779
Noninferior Proportion Difference - Unstratified CMH Method (95% CI)	0.04 (-0.21, 0.29)		
Superior Proportion Difference - 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

1.1.2 Post-hoc

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)	

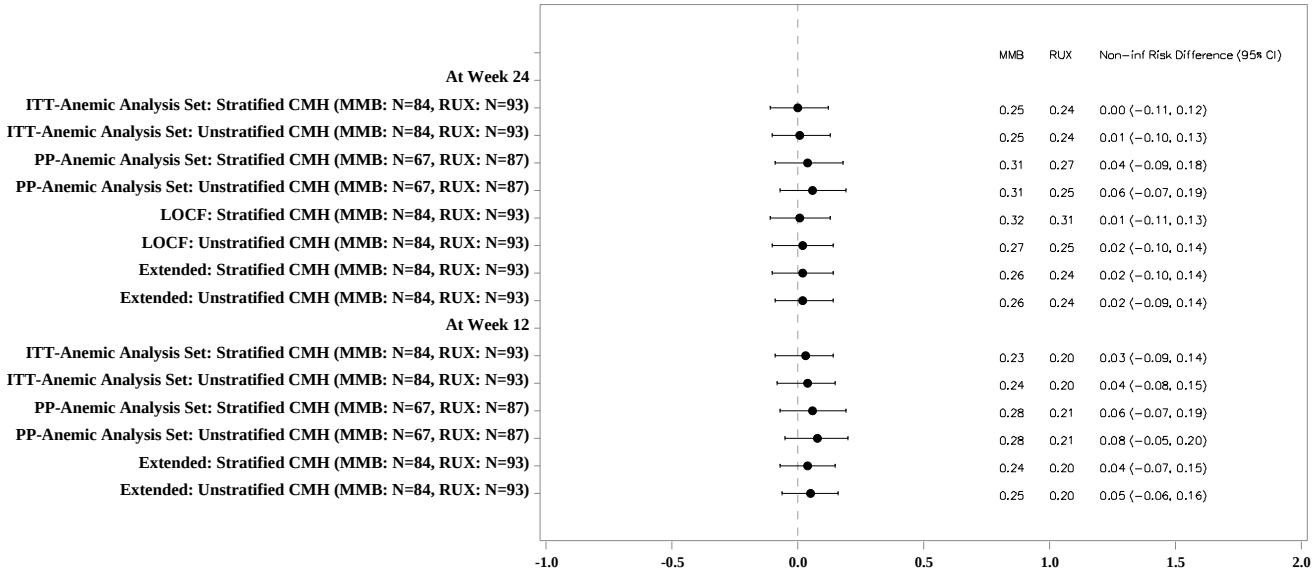
ITT-Anemic Analysis includes subjects in the Intent-to-Treat Population with Baseline Hemoglobin < 10 g/dL.
Stratified two-sided p-value is from a log-rank test comparing treatment stratified by baseline TD (yes,no) and baseline platelet count (<100,100-200,>200 10^9/L). Adjusted hazard ratio and 95% 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

1.2 Symptomansprechen

GSK Oncology
Study GS-US-352-0101

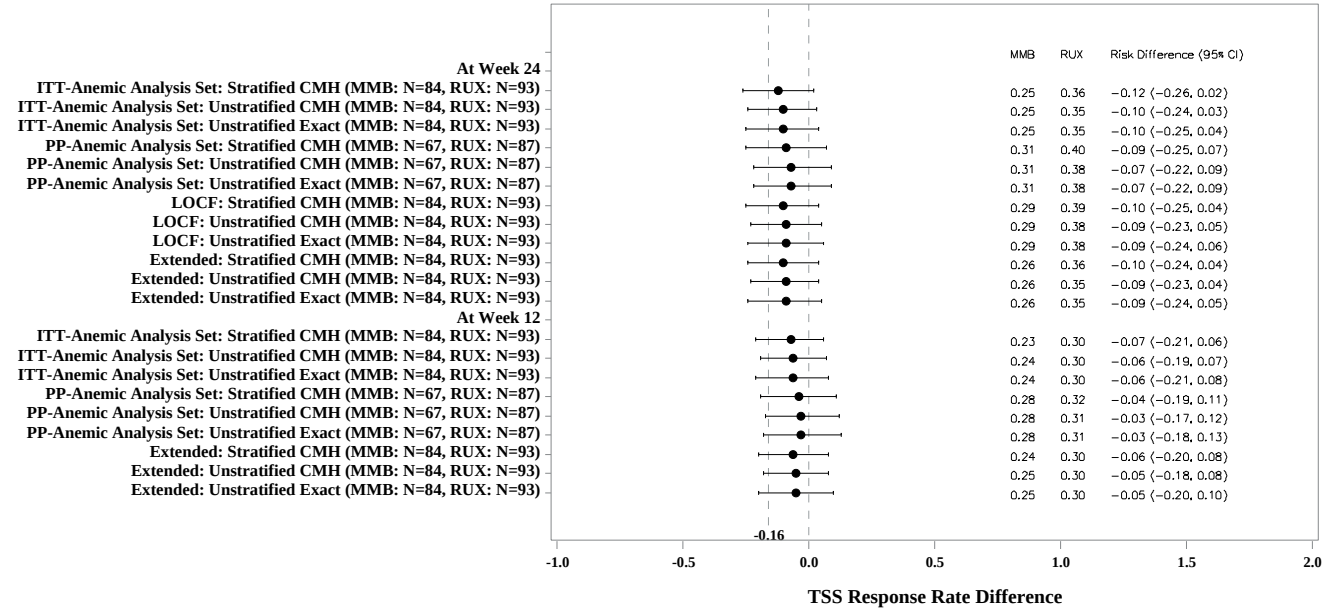
Figure 2.1001: Forest plot of Primary and Sensitivity Analysis of Response Rate in TSS at Week 24 and Week 12
Double-Blind Phase
ITT-Anemic and PP-Anemic Analysis Sets
Noninferiority



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
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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 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
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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	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
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	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;

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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	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
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 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
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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 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
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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 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
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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)
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

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

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

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

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

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

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

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

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

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

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
Source: t-tss24-sen.sas V.03.05 Output file: t-sen-ppt-tss24.pdf 29AUG2023: 9:45

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
Source: t-tss24-sen.sas V.03.05 Output file: t-sen-ppt-tss24.pdf 29AUG2023: 9:45

GSK Oncology

Study GS-US-352-0101

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

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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)
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

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.
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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)
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

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.
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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)
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.
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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)
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.
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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)
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.
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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)
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.
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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)
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

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.
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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)
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.
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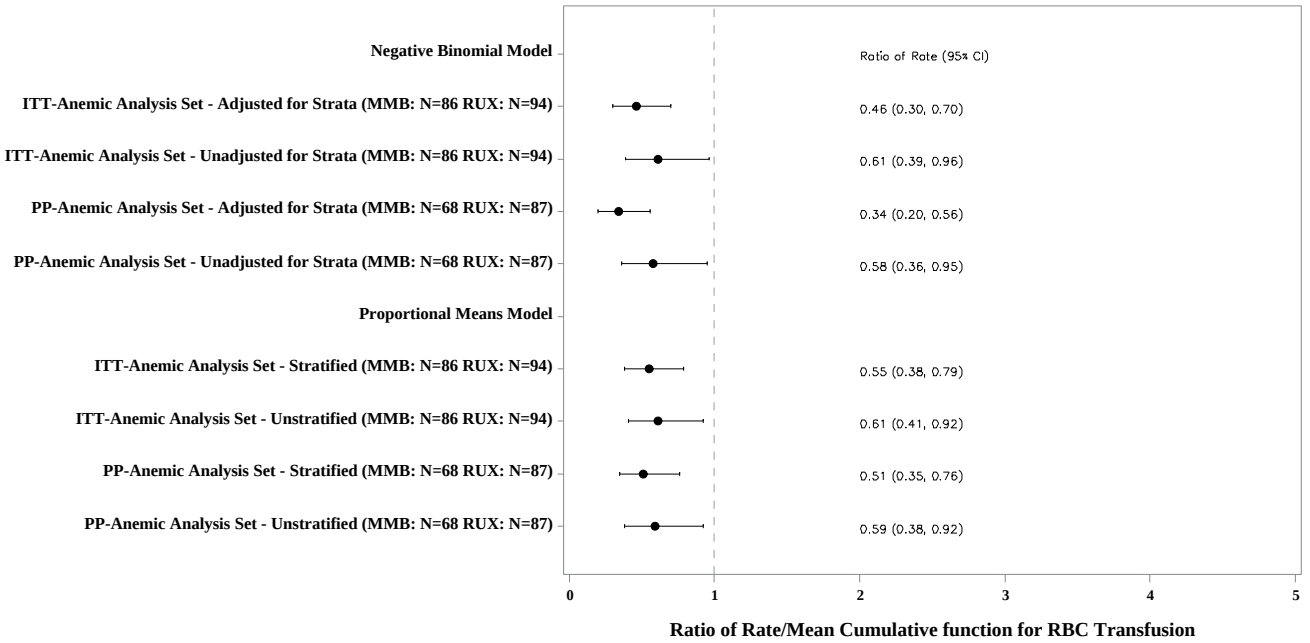
1.3 Transfusionsbezogene Endpunkte

1.3.1 Pre-defined

1.3.1.1 TI

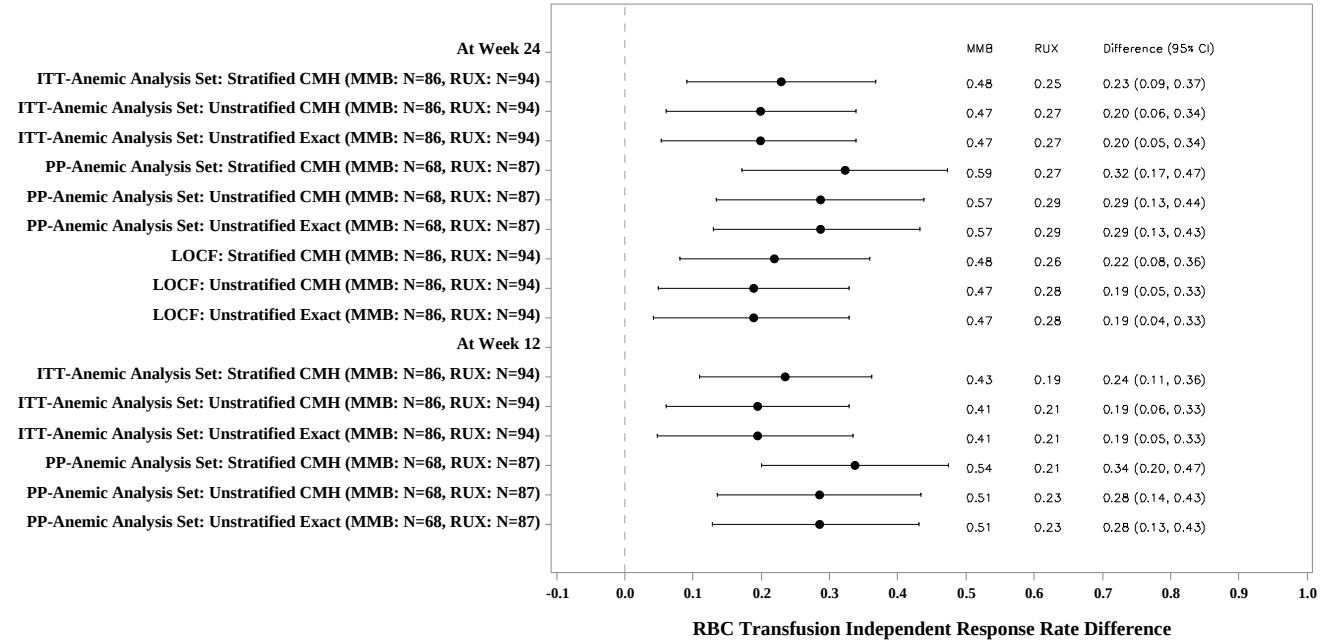
Figure 2.1301: Forest plot of Primary and Sensitivity Analysis of Rate of RBC Transfusion
Double-Blind Phase
ITT-Anemic and PP-Anemic Analysis Sets
All strata combined



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

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
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(%)	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

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

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

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

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.

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)
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.

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(%)	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

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.

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)
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
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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(%)	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.

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 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
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(%)	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

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.

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
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(%)	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.

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.

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.

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.

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.

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.

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.

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
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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)
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

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

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

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

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

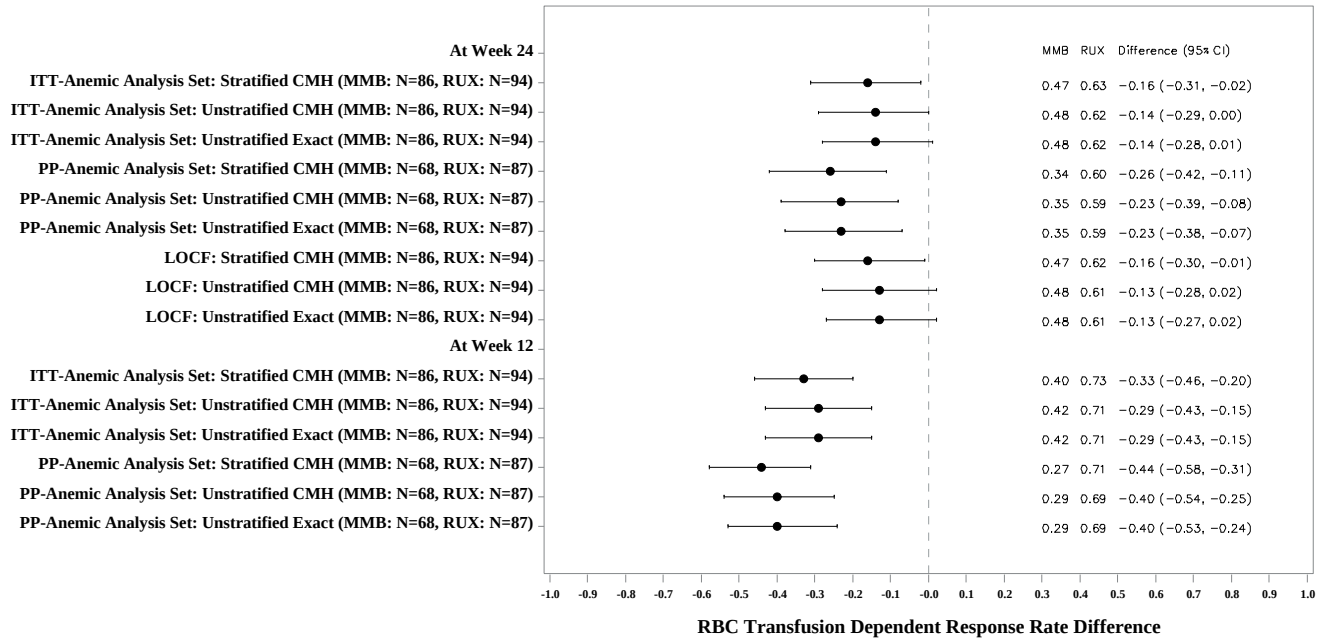
	MMB (N=68)	RUX (N=87)	Total (N=155)
Duration of the DB phase (months) in stratum 6			
N	14	31	45
Mean (SD)	5.50 (0.236)	5.58 (0.101)	5.56 (0.157)
Median	5.55	5.55	5.55
Q1, Q3	5.45, 5.59	5.52, 5.62	5.52, 5.62
Min, Max	4.80, 5.78	5.36, 5.78	4.80, 5.78

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

1.3.1.2 TD

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
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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)
>=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.
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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(%)	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.
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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)
>=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.
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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)
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.
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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(%)	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.
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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)
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.
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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(%)	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.
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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)
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

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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(%)	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

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

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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(%)	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
Source: t-rbctd24-gba.sas V.03.05 Output file: t-rbctd24-gba.pdf 29AUG2023: 9:36

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

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

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

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

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

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

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

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

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

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

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%)

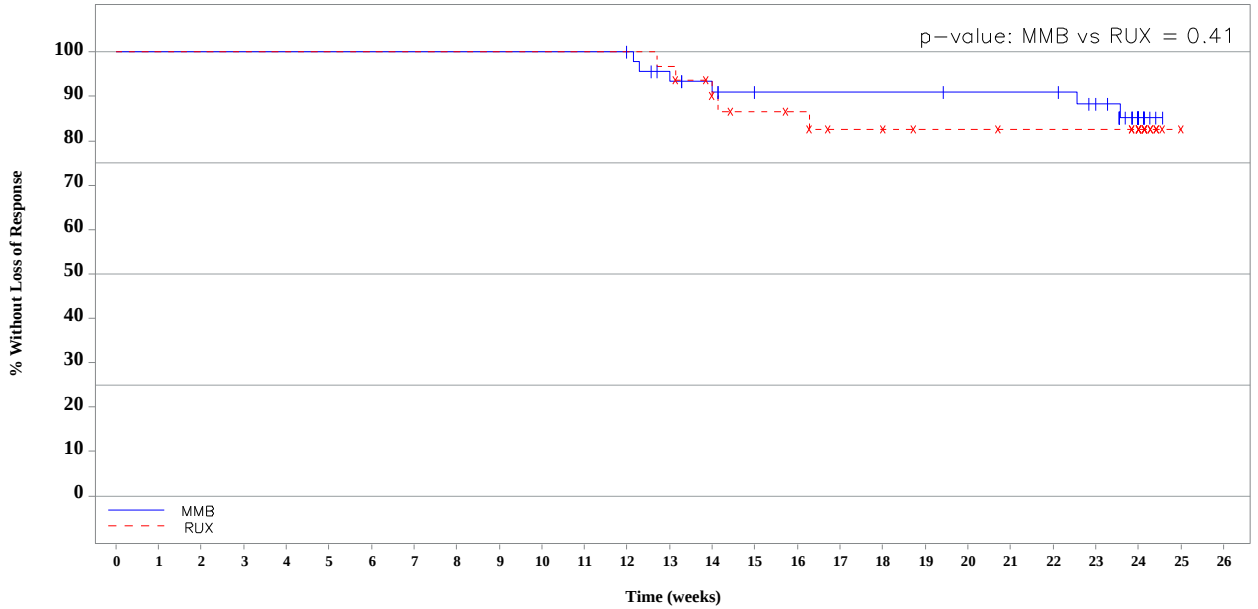
PP-Anemic Analysis includes subjects in the Per-Protocol Population with Baseline Hemoglobin < 10 g/dL.
95% Exact CI is based on Clopper-Pearson method without stratification.
Transfusion requiring is defined as being neither transfusion independent nor transfusion dependent.
CMH = Cochran-Mantel-Haenszel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

Data Extracted: CRF data: 01JUL2019

1.3.2 Post-hoc

1.3.2.1 TI

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)	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

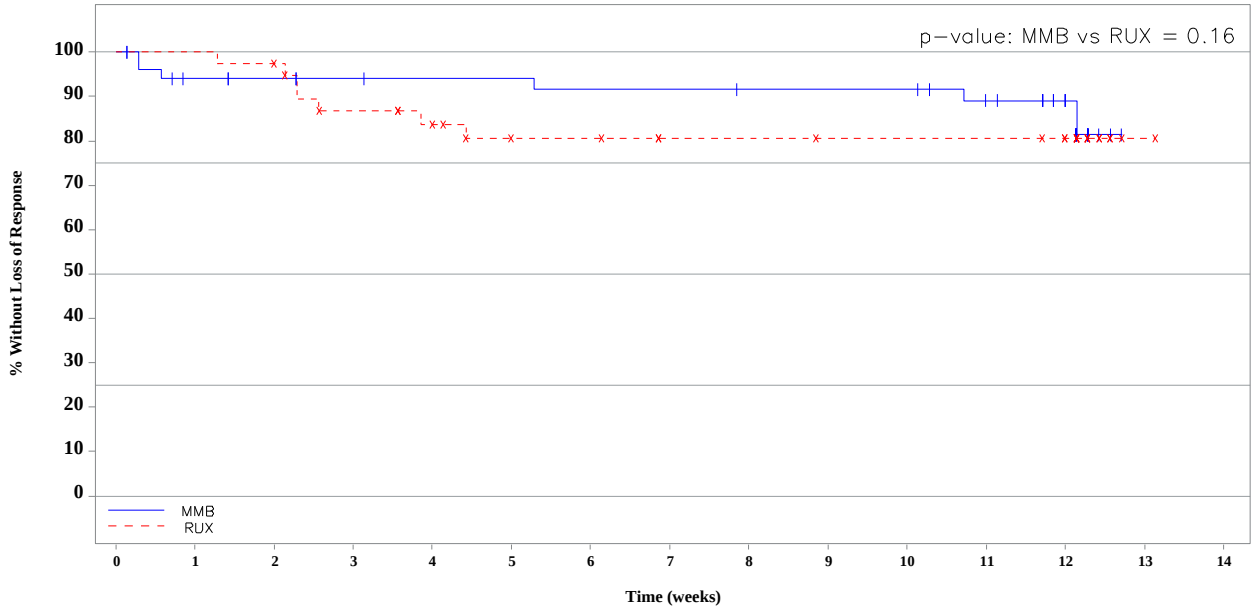
Table 2.3701: Analysis of Duration of RBC Transfusion Independent Response
Double-blind Phase
ITT-Anemic Analysis Set With RBC TI Response

	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

1.3.2.2 TF

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



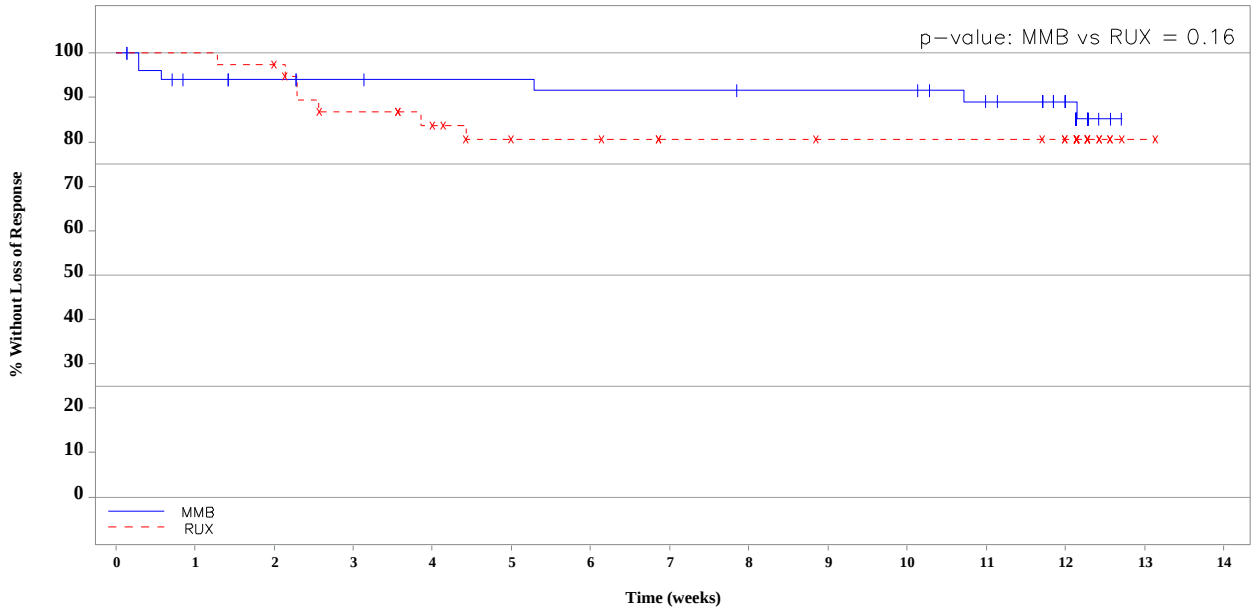
N at Risk (Events)

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

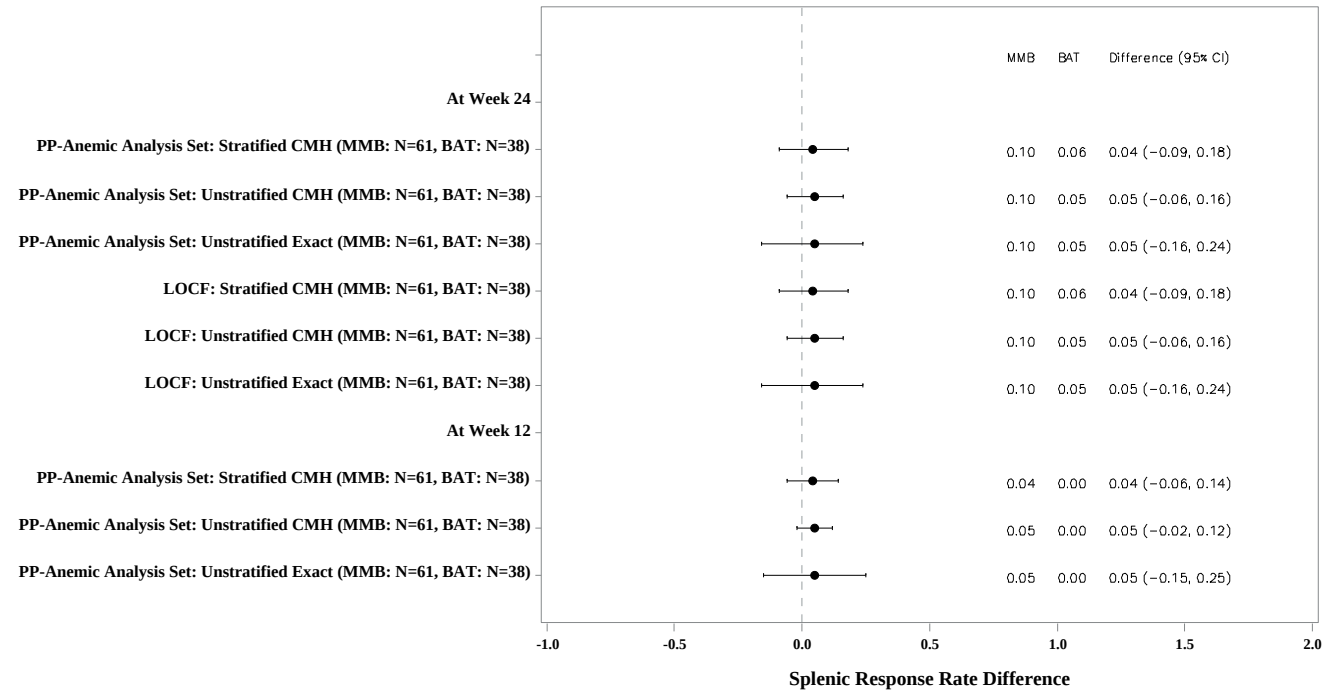
2 SIMPLIFY-2

2.1 Milzansprechen

2.1.1 Pre-defined

GSK Oncology
Study GS-US-352-1214

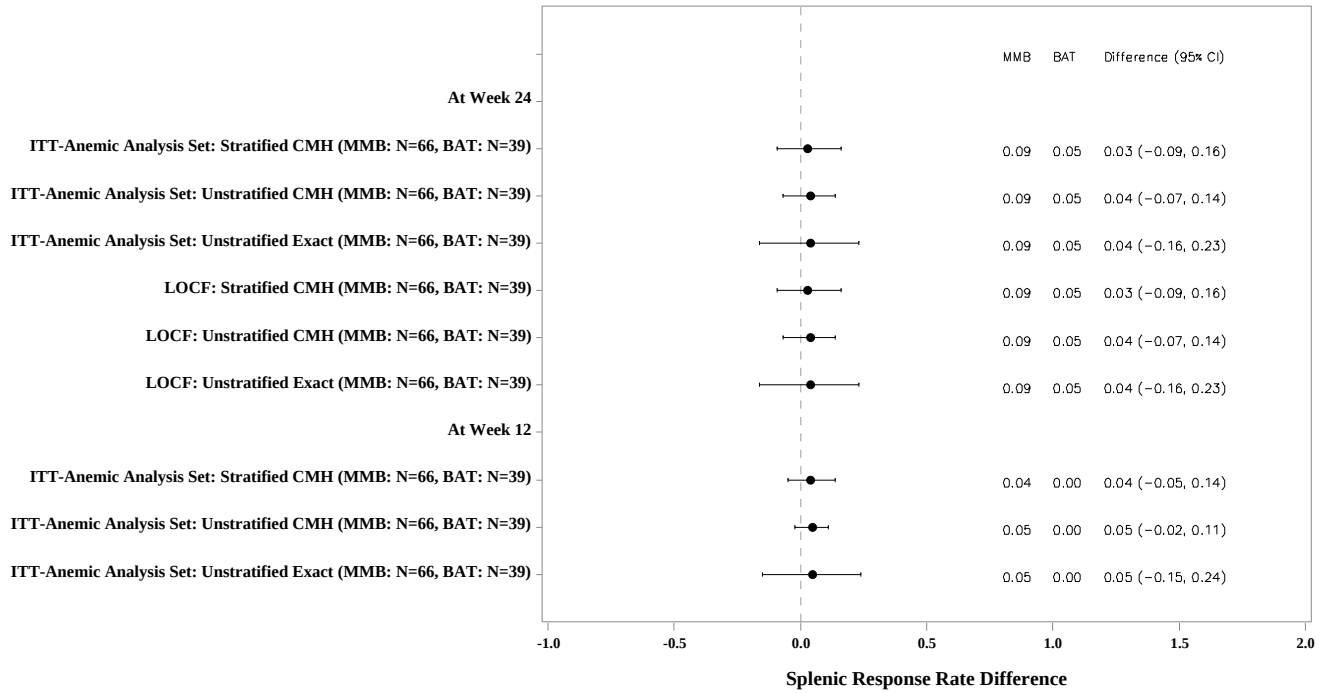
Figure 2.0202: Forest Plot of Primary and Sensitivity Analysis of Splenic Response Rate at Week 24 and Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set
All strata combined



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

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

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
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(%)	**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.

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.

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.

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.

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.

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.

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.

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.

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.

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
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(%)	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

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

GSK Oncology
Study GS-US-352-1214

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

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

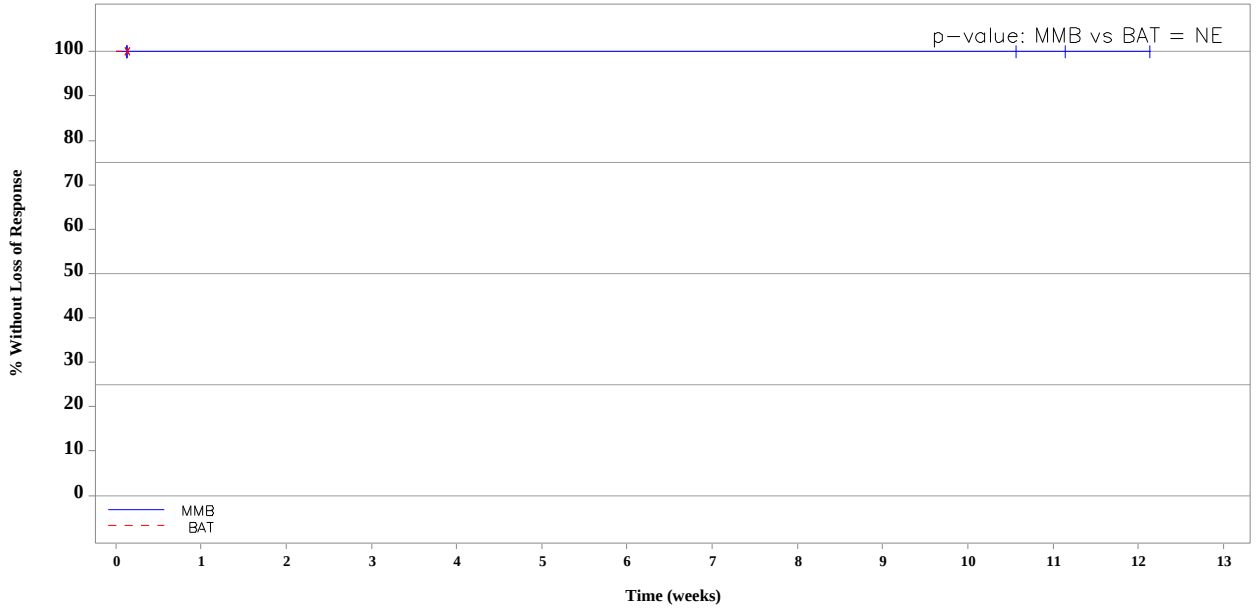
	MMB (N=66)	BAT (N=39)	Total (N=105)
Splenic Response Rate at Week 24			
Responder, n(%)	1 (14.3%)	0	1 (6.7%)
95% Exact CI	0.0036, 0.5787	0.0000, 0.3694	0.0017, 0.3195
Proportion Difference (95% CI)	0.14 (-0.21, 0.50)		
Proportion Difference using Exact Method (95% CI)	0.14 (-0.36, 0.58)		
Non-Responder, 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

2.1.2 Post-hoc

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

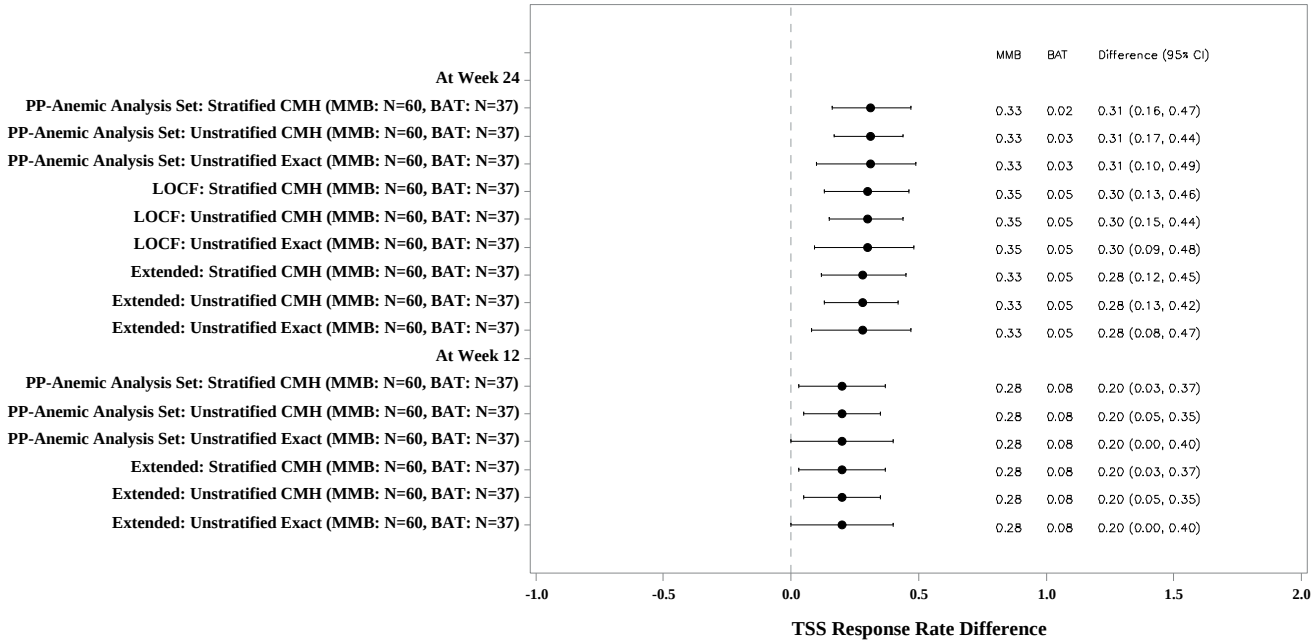
	MMB (N=6)	BAT (N=2)
Subjects with Event		
Loss of spleen response, n(%)	0	0
Censor		
Subjects Censored, n(%)	6 (100.0%)	2 (100.0%)
Non-Responder: Censored at Randomization Date	0	0
No Loss of Response: Censored at Last MRI Spleen Assessment Date in RT phase (including up to 10 days after First Dose Date 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

2.2 Symptomansprechen

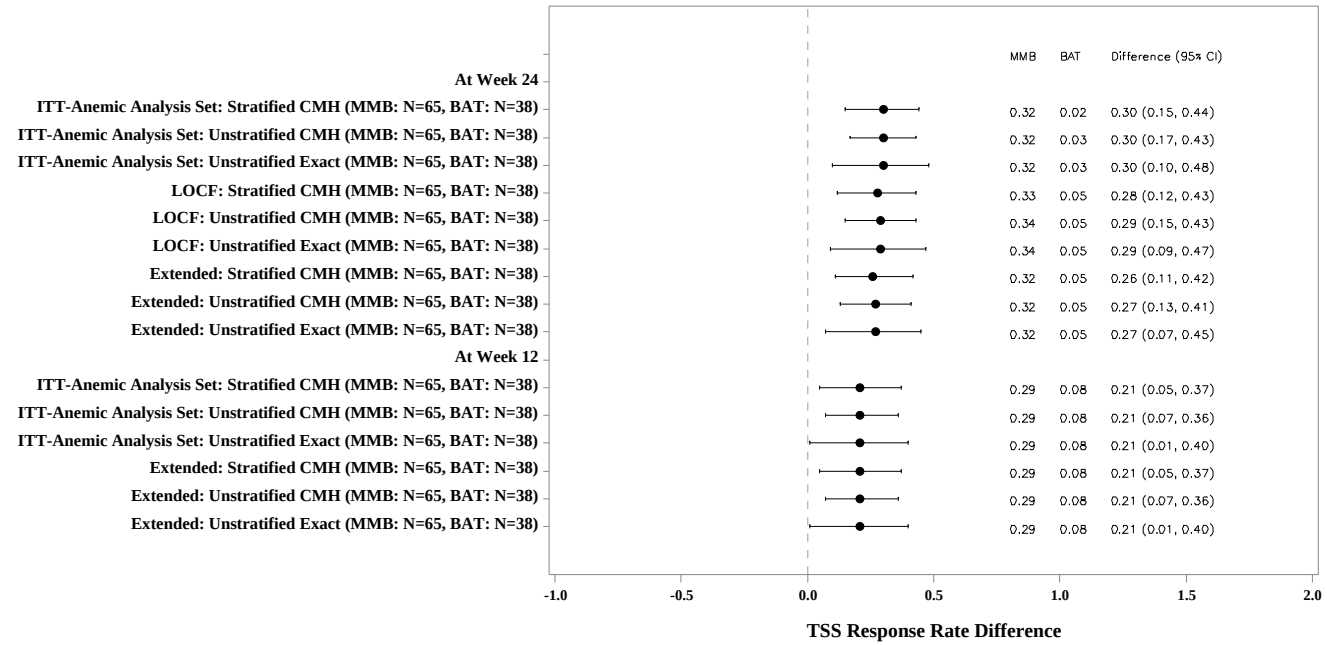
Figure 2.1302: Forest plot of Primary and Sensitivity Analysis of Response Rate in TSS at Week 24 and Week 12
Randomized Treatment Phase
PP-Anemic Analysis Set
All strata combined



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

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

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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

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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

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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

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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

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

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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

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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

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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

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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;

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;

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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
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	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

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

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

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.

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

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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

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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

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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

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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

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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

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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

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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

2.3 Transfusionsbezogene Endpunkte

2.3.1 Pre-defined

2.3.1.1 TI

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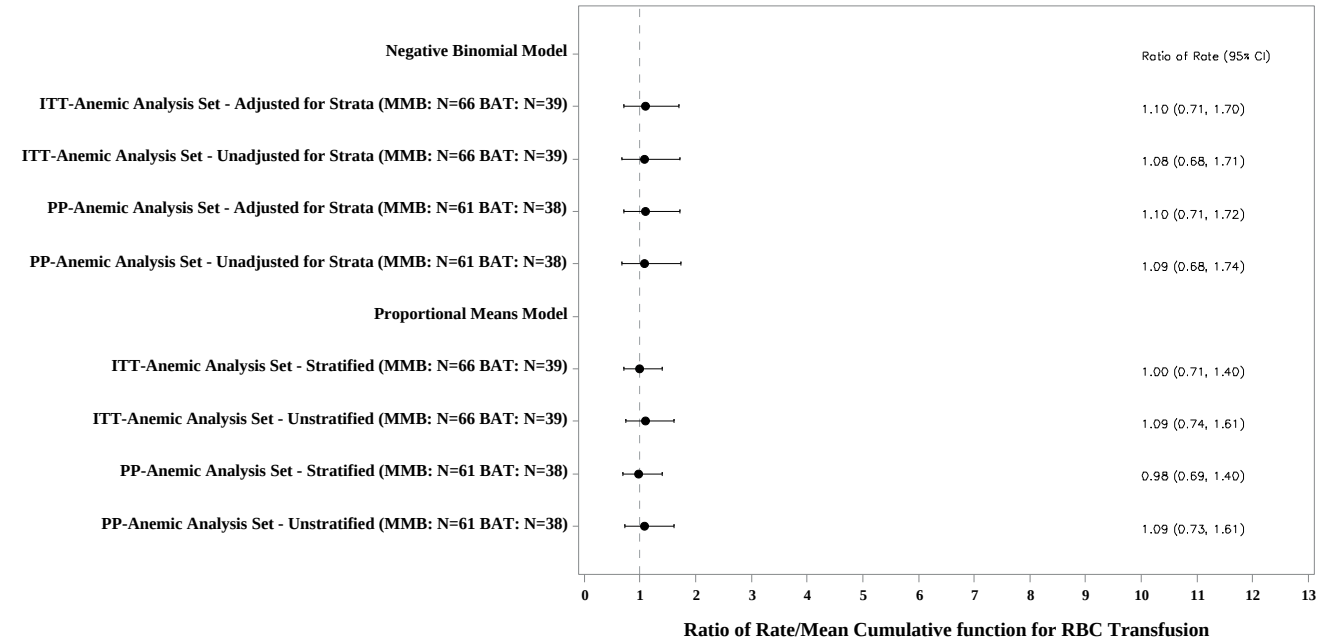
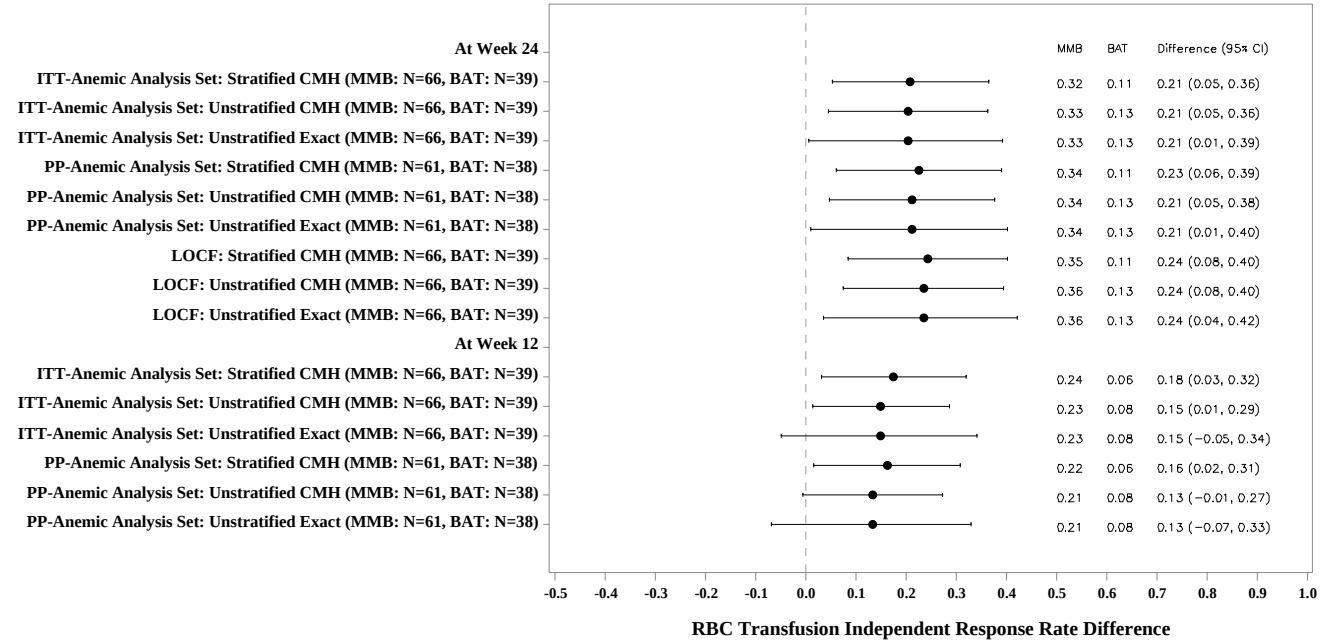
Figure 2.1401: Forest Plot of Primary and Sensitivity Analysis of Rate of RBC Transfusion
Randomized Treatment Phase
ITT-Anemic Analysis Set
All strata combined



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

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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

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.

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.

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.

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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

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.

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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.

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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.

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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

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

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

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

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

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

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

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
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)
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

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

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

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

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

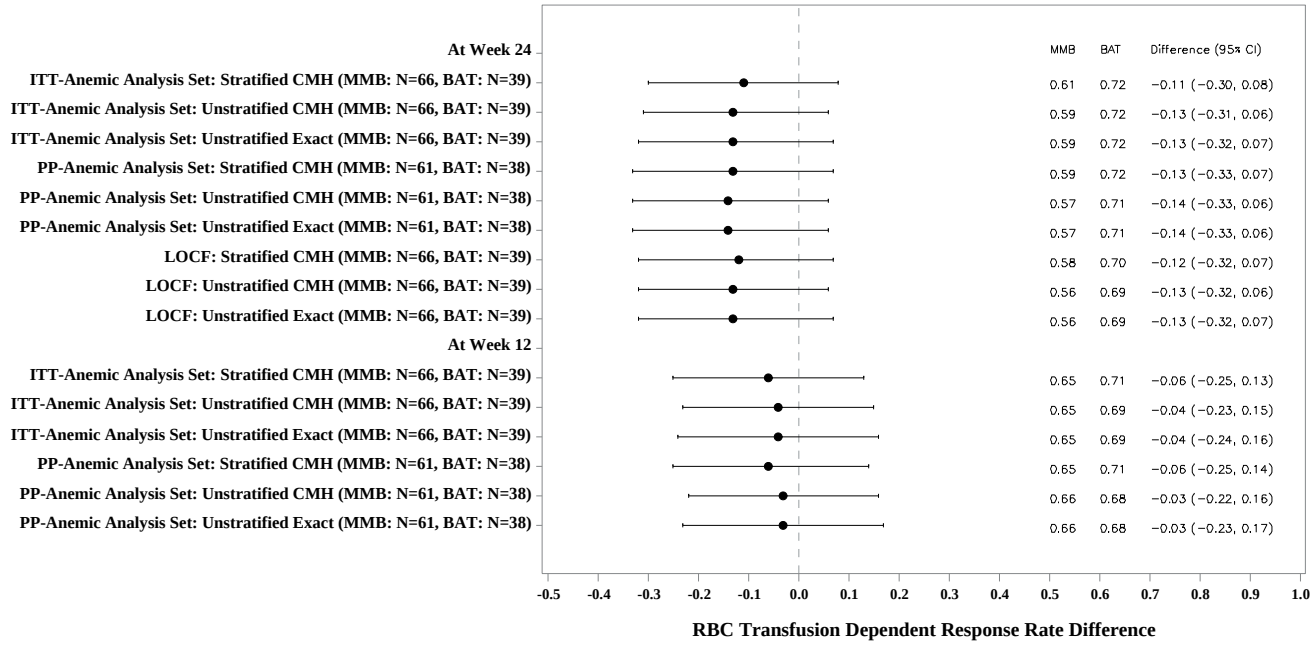
Table 2.2701: Sensitivity Analysis of Rate of RBC Transfusion
Randomized Treatment Phase
PP-Anemic Analysis Set

	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

2.3.1.2 TD

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

GSK Oncology

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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

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
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)
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.

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.

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.

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.

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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

GSK Oncology
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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

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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

GSK Oncology
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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
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)
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.

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
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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.

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
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 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

2.3.2 Post-hoc

2.3.2.1 TI

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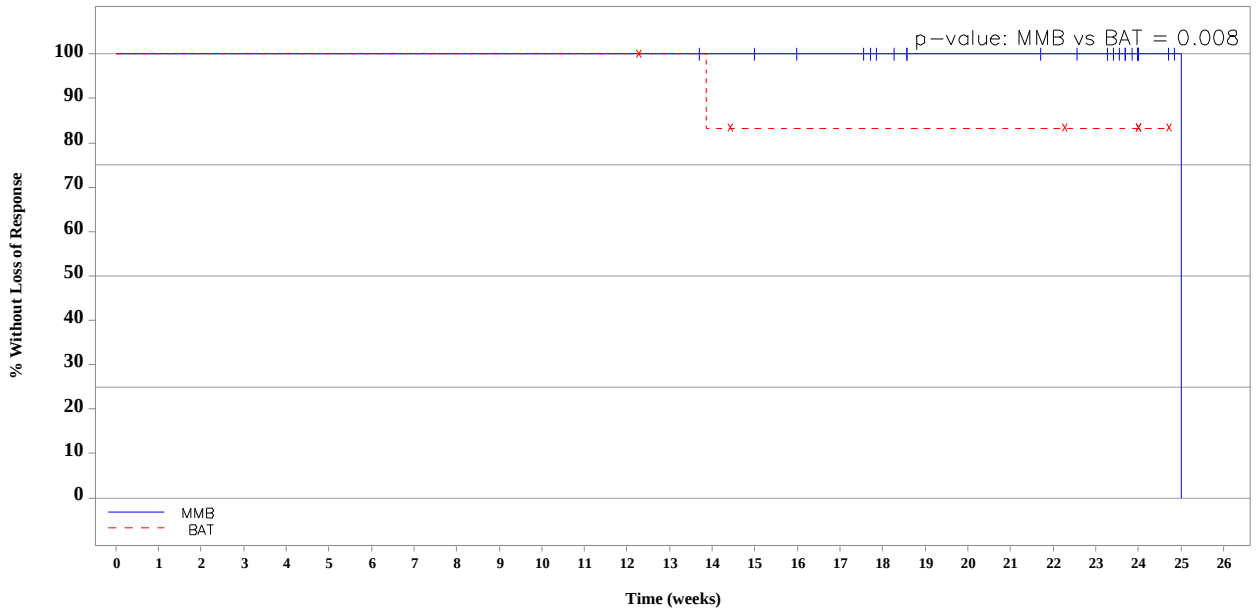
Figure 2.2201: Kaplan-Meier Plot of Duration of RBC Transfusion Independent Response
Randomized Treatment Phase
ITT-Anemic Analysis Set With RBC Transfusion Independent Response



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

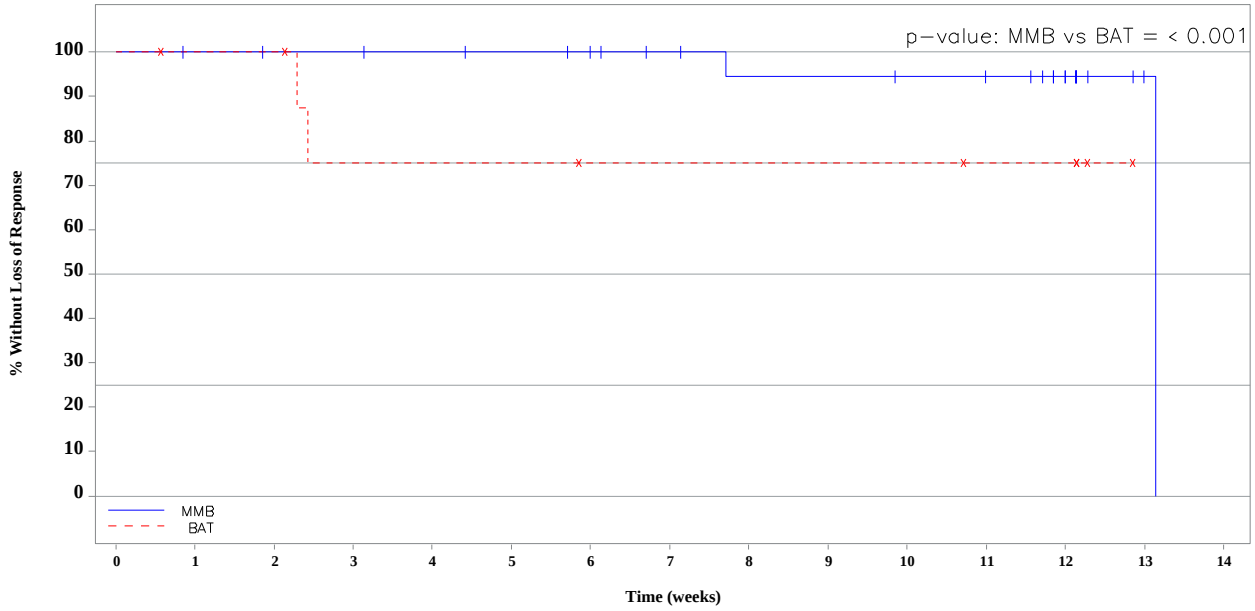
	MMB (N=25)	BAT (N=7)
Subjects with Event		
Loss of RBC Transfusion Independent Response, n(%)	1 (4.0%)	1 (14.3%)
Censor		
Subjects Censored, n(%)	24 (96.0%)	6 (85.7%)
No Loss of Response: Censored at Last Subject Visit Date at RT Phase	24 (100.0%)	6 (100.0%)
Kaplan-Meier Estimate of Duration of RBC Transfusion 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

2.3.2.2 TF

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



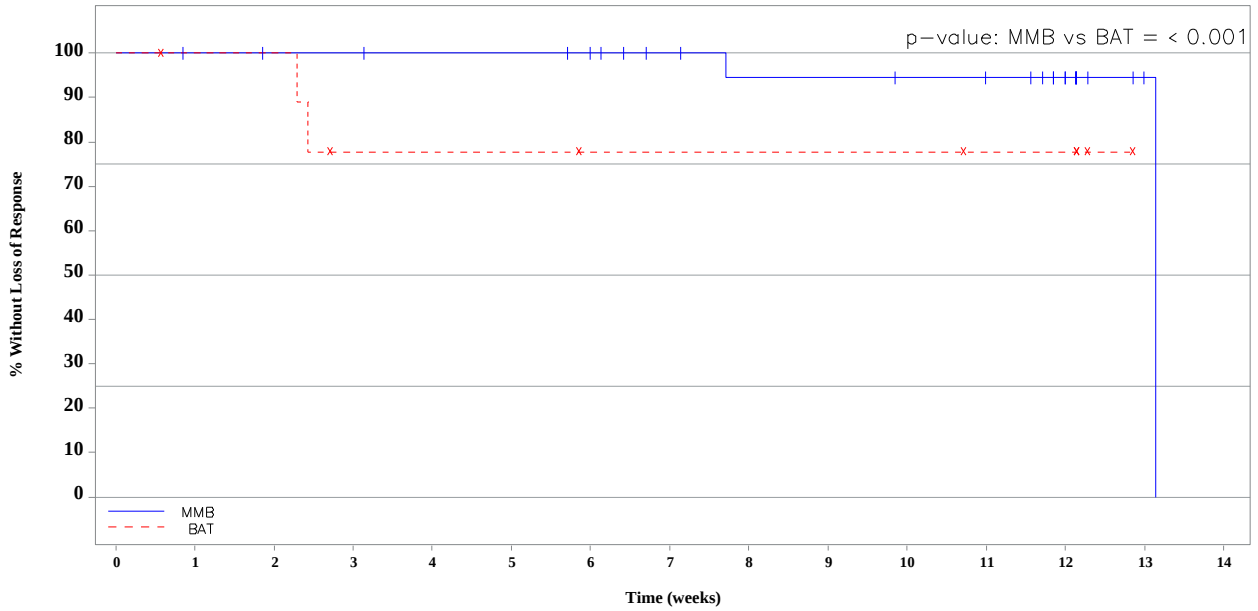
N at Risk (Events)

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.

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