

Table 15.12.3.1: Analysis of Rate of RBC Transfusion
Double-Blind Phase
Intent-to-Treat Analysis Set

	MMB (N=215)	RUX (N=217)	Total (N=432)
All Strata Combined			
RBC transfusion Rate in DB phase (units/month)			
N	215	217	432
Mean (SD)	0.5 (1.27)	1.0 (1.62)	0.8 (1.48)
Median	0.0	0.4	0.0
Q1, Q3	0.0, 0.2	0.0, 1.5	0.0, 0.9
Min, Max	0.0, 9.1	0.0, 8.2	0.0, 9.1
Negative Binomial Model			
Adjusted for Strata			
Rate of RBC transfusion with 95% CI (units/month)	0.43 (0.31, 0.59)	1.51 (1.06, 2.15)	
Ratio of Rate for RBC Transfusion with 95% CI	0.28 (0.19, 0.43)	-	-
p-value	< 0.001	-	-
Unadjusted for Strata			
Rate of RBC transfusion with 95% CI (units/month)	0.50 (0.36, 0.70)	1.03 (0.75, 1.42)	
Ratio of Rate for RBC Transfusion with 95% CI	0.49 (0.31, 0.78)	-	-
p-value	0.002	-	-
Proportional Means Model - Supportive Analysis			
Stratified			
Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI	0.46 (0.33, 0.65)	-	-
p-value	< 0.001	-	-
Unstratified			
Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI	0.47 (0.32, 0.71)	-	-
p-value	< 0.001	-	-

Proportional means model is Andersen-Gill model with robust sandwich variance estimate.

RBC = Red Blood Cell

Source: Listing 16.3.3.1.1

Data Extracted: CRF data: 01JUL2019

Source: ...\\version5\prog\t-rbc24.sas v9.4 Output file: t-rbc24.out 09MAR2021:17:32

Table 15.12.3.1: Analysis of Rate of RBC Transfusion
Double-Blind Phase
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	MMB (N=215)	RUX (N=217)	Total (N=432)
Total number of RBC transfusion unit in DB phase			
N	215	217	432
Mean (SD)	2.4 (6.21)	5.4 (8.23)	3.9 (7.44)
Median	0.0	2.0	0.0
Q1, Q3	0.0, 1.0	0.0, 8.0	0.0, 4.0
Min, Max	0.0, 47.0	0.0, 44.0	0.0, 47.0
Duration of the DB phase (months)			
N	215	217	432
Mean (SD)	5.11 (1.274)	5.45 (0.608)	5.28 (1.010)
Median	5.55	5.55	5.55
Q1, Q3	5.45, 5.59	5.52, 5.59	5.49, 5.59
Min, Max	0.03, 5.78	0.30, 5.78	0.03, 5.78

Proportional means model is Andersen-Gill model with robust sandwich variance estimate.

RBC = Red Blood Cell

Source: Listing 16.3.3.1.1

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Table 15.12.3.1: Analysis of Rate of RBC Transfusion
Double-Blind Phase
Intent-to-Treat Analysis Set

	MMB (N=215)	RUX (N=217)	Total (N=432)
Stratum 1			
Transfusion Dependence at Baseline = 'Yes' and Platelet Count < 100*10E9/L			
RBC transfusion Rate in DB phase (units/month)			
N	10	11	21
Mean (SD)	1.8 (1.60)	3.4 (2.88)	2.7 (2.44)
Median	1.5	3.2	2.9
Q1, Q3	0.4, 3.3	0.4, 5.2	0.4, 3.5
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)	1.82 (0.93, 3.57)	3.39 (1.81, 6.35)	
Ratio of Rate for RBC Transfusion with 95% CI	0.54 (0.21, 1.34)	-	-
Proportional Means Model - Supportive Analysis			
Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI	0.56 (0.27, 1.16)	-	-
Total number of RBC transfusion in stratum 1 in DB phase			
N	10	11	21
Mean (SD)	9.0 (8.49)	15.8 (12.91)	12.6 (11.31)
Median	6.5	16.0	9.0
Q1, Q3	2.0, 18.0	2.0, 27.0	2.0, 19.0
Min, Max	0.0, 24.0	0.0, 38.0	0.0, 38.0

Proportional means model is Andersen-Gill model with robust sandwich variance estimate.

RBC = Red Blood Cell

Source: Listing 16.3.3.1.1

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Table 15.12.3.1: Analysis of Rate of RBC Transfusion
Double-Blind Phase
Intent-to-Treat Analysis Set

	MMB (N=215)	RUX (N=217)	Total (N=432)
Duration of the DB phase (months) in stratum 1			
N	10	11	21
Mean (SD)	5.18 (1.174)	5.01 (1.045)	5.09 (1.084)
Median	5.55	5.55	5.55
Q1, Q3	5.49, 5.59	4.63, 5.55	5.49, 5.55
Min, Max	1.84, 5.62	2.56, 5.62	1.84, 5.62

Proportional means model is Andersen-Gill model with robust sandwich variance estimate.

RBC = Red Blood Cell

Source: Listing 16.3.3.1.1

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Table 15.12.3.1: Analysis of Rate of RBC Transfusion
Double-Blind Phase
Intent-to-Treat Analysis Set

	MMB (N=215)	RUX (N=217)	Total (N=432)
Stratum 2			
Transfusion Dependence at Baseline = 'Yes' and Platelet Count $\geq 100 \times 10^9/L$ and $\leq 200 \times 10^9/L$			
RBC transfusion Rate in DB phase (units/month)			
N	21	20	41
Mean (SD)	1.9 (2.40)	2.3 (2.29)	2.1 (2.33)
Median	1.3	1.3	1.3
Q1, Q3	0.0, 2.0	0.6, 4.2	0.4, 3.6
Min, Max	0.0, 9.1	0.0, 6.9	0.0, 9.1
Negative Binomial Model			
Rate of RBC transfusion with 95% CI (units/month)	1.88 (1.07, 3.31)	2.31 (1.31, 4.09)	
Ratio of Rate for RBC Transfusion with 95% CI	0.81 (0.36, 1.82)	-	-
Proportional Means Model - Supportive Analysis			
Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI	0.84 (0.41, 1.74)	-	-
Total number of RBC transfusion in stratum 2 in DB phase			
N	21	20	41
Mean (SD)	9.0 (12.85)	11.0 (10.82)	9.9 (11.80)
Median	4.0	7.0	4.0
Q1, Q3	0.0, 8.0	3.5, 19.0	2.0, 13.0
Min, Max	0.0, 47.0	0.0, 38.0	0.0, 47.0

Proportional means model is Andersen-Gill model with robust sandwich variance estimate.

RBC = Red Blood Cell

Source: Listing 16.3.3.1.1

Data Extracted: CRF data: 01JUL2019

Source: ...\\version5\prog\t-rbc24.sas v9.4 Output file: t-rbc24.out 09MAR2021:17:32

Table 15.12.3.1: Analysis of Rate of RBC Transfusion
Double-Blind Phase
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	MMB (N=215)	RUX (N=217)	Total (N=432)
Duration of the DB phase (months) in stratum 2			
N	21	20	41
Mean (SD)	4.79 (1.696)	4.95 (1.353)	4.87 (1.522)
Median	5.55	5.50	5.55
Q1, Q3	5.52, 5.59	5.32, 5.55	5.36, 5.55
Min, Max	0.53, 5.65	0.30, 5.68	0.30, 5.68

Proportional means model is Andersen-Gill model with robust sandwich variance estimate.

RBC = Red Blood Cell

Source: Listing 16.3.3.1.1

Data Extracted: CRF data: 01JUL2019

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Table 15.12.3.1: Analysis of Rate of RBC Transfusion
Double-Blind Phase
Intent-to-Treat Analysis Set

	MMB (N=215)	RUX (N=217)	Total (N=432)
Stratum 3			
Transfusion Dependence at Baseline = 'Yes' and Platelet Count > 200*10E9/L			
RBC transfusion Rate in DB phase (units/month)			
N	22	21	43
Mean (SD)	1.3 (1.27)	2.5 (2.06)	1.9 (1.78)
Median	0.8	1.8	1.5
Q1, Q3	0.2, 2.5	0.9, 4.0	0.4, 2.9
Min, Max	0.0, 3.6	0.0, 7.8	0.0, 7.8
Negative Binomial Model			
Rate of RBC transfusion with 95% CI (units/month)	1.35 (0.86, 2.13)	2.46 (1.58, 3.83)	
Ratio of Rate for RBC Transfusion with 95% CI	0.55 (0.29, 1.04)	-	-
Proportional Means Model - Supportive Analysis			
Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI	0.55 (0.32, 0.94)	-	-
Total number of RBC transfusion in stratum 3 in DB phase			
N	22	21	43
Mean (SD)	6.8 (7.10)	13.7 (11.56)	10.1 (10.05)
Median	3.5	10.0	7.0
Q1, Q3	1.0, 14.0	5.0, 22.0	2.0, 16.0
Min, Max	0.0, 20.0	0.0, 44.0	0.0, 44.0

Proportional means model is Andersen-Gill model with robust sandwich variance estimate.

RBC = Red Blood Cell

Source: Listing 16.3.3.1.1

Data Extracted: CRF data: 01JUL2019

Source: ...\\version5\\prog\\t-rbc24.sas v9.4 Output file: t-rbc24.out 09MAR2021:17:32

Table 15.12.3.1: Analysis of Rate of RBC Transfusion
Double-Blind Phase
Intent-to-Treat Analysis Set

	MMB (N=215)	RUX (N=217)	Total (N=432)
Duration of the DB phase (months) in stratum 3			
N	22	21	43
Mean (SD)	5.02 (1.455)	5.55 (0.090)	5.28 (1.065)
Median	5.55	5.55	5.55
Q1, Q3	5.52, 5.59	5.52, 5.59	5.52, 5.59
Min, Max	0.30, 5.78	5.32, 5.78	0.30, 5.78

Proportional means model is Andersen-Gill model with robust sandwich variance estimate.

RBC = Red Blood Cell

Source: Listing 16.3.3.1.1

Data Extracted: CRF data: 01JUL2019

Source: ...\\version5\\prog\\t-rbc24.sas v9.4 Output file: t-rbc24.out 09MAR2021:17:32

Table 15.12.3.1: Analysis of Rate of RBC Transfusion
Double-Blind Phase
Intent-to-Treat Analysis Set

	MMB (N=215)	RUX (N=217)	Total (N=432)
Stratum 4			
Transfusion Dependence at Baseline = 'No' and Platelet Count < 100*10E9/L			
RBC transfusion Rate in DB phase (units/month)			
N	8	12	20
Mean (SD)	0.3 (0.94)	0.4 (0.72)	0.4 (0.79)
Median	0.0	0.0	0.0
Q1, Q3	0.0, 0.0	0.0, 0.4	0.0, 0.1
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)	0.34 (0.04, 3.16)	0.37 (0.06, 2.26)	
Ratio of Rate for RBC Transfusion with 95% CI	0.92 (0.05, 16.21)	-	-
Proportional Means Model - Supportive Analysis			
Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI	1.00 (0.13, 7.96)	-	-
Total number of RBC transfusion in stratum 4 in DB phase			
N	8	12	20
Mean (SD)	1.9 (5.30)	2.1 (4.08)	2.0 (4.47)
Median	0.0	0.0	0.0
Q1, Q3	0.0, 0.0	0.0, 2.0	0.0, 0.5
Min, Max	0.0, 15.0	0.0, 12.0	0.0, 15.0

Proportional means model is Andersen-Gill model with robust sandwich variance estimate.

RBC = Red Blood Cell

Source: Listing 16.3.3.1.1

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Table 15.12.3.1: Analysis of Rate of RBC Transfusion
Double-Blind Phase
Intent-to-Treat Analysis Set

	MMB (N=215)	RUX (N=217)	Total (N=432)
Duration of the DB phase (months) in stratum 4			
N	8	12	20
Mean (SD)	4.95 (1.658)	5.51 (0.246)	5.29 (1.062)
Median	5.52	5.55	5.55
Q1, Q3	5.44, 5.60	5.49, 5.62	5.47, 5.60
Min, Max	0.85, 5.65	4.80, 5.78	0.85, 5.78

Proportional means model is Andersen-Gill model with robust sandwich variance estimate.

RBC = Red Blood Cell

Source: Listing 16.3.3.1.1

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Table 15.12.3.1: Analysis of Rate of RBC Transfusion
Double-Blind Phase
Intent-to-Treat Analysis Set

	MMB (N=215)	RUX (N=217)	Total (N=432)
Stratum 5			
Transfusion Dependence at Baseline = 'No' and Platelet Count $\geq 100 \times 10^9/L$ and $\leq 200 \times 10^9/L$			
RBC transfusion Rate in DB phase (units/month)			
N	45	43	88
Mean (SD)	0.2 (1.10)	0.6 (0.78)	0.4 (0.96)
Median	0.0	0.2	0.0
Q1, Q3	0.0, 0.0	0.0, 1.1	0.0, 0.4
Min, Max	0.0, 6.8	0.0, 3.2	0.0, 6.8
Negative Binomial Model			
Rate of RBC transfusion with 95% CI (units/month)	0.13 (0.05, 0.32)	0.56 (0.26, 1.19)	
Ratio of Rate for RBC Transfusion with 95% CI	0.23 (0.07, 0.75)	-	-
Proportional Means Model - Supportive Analysis			
Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI	0.19 (0.05, 0.76)	-	-
Total number of RBC transfusion in stratum 5 in DB phase			
N	45	43	88
Mean (SD)	0.6 (2.54)	3.1 (4.32)	1.8 (3.72)
Median	0.0	1.0	0.0
Q1, Q3	0.0, 0.0	0.0, 6.0	0.0, 2.0
Min, Max	0.0, 16.0	0.0, 18.0	0.0, 18.0

Proportional means model is Andersen-Gill model with robust sandwich variance estimate.

RBC = Red Blood Cell

Source: Listing 16.3.3.1.1

Data Extracted: CRF data: 01JUL2019

Source: ...\\version5\prog\t-rbc24.sas v9.4 Output file: t-rbc24.out 09MAR2021:17:32

Table 15.12.3.1: Analysis of Rate of RBC Transfusion
Double-Blind Phase
Intent-to-Treat Analysis Set

	MMB (N=215)	RUX (N=217)	Total (N=432)
Duration of the DB phase (months) in stratum 5			
N	45	43	88
Mean (SD)	5.30 (0.901)	5.51 (0.399)	5.40 (0.706)
Median	5.55	5.55	5.55
Q1, Q3	5.45, 5.59	5.52, 5.59	5.49, 5.59
Min, Max	0.30, 5.65	3.06, 5.78	0.30, 5.78

Proportional means model is Andersen-Gill model with robust sandwich variance estimate.

RBC = Red Blood Cell

Source: Listing 16.3.3.1.1

Data Extracted: CRF data: 01JUL2019

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Table 15.12.3.1: Analysis of Rate of RBC Transfusion
Double-Blind Phase
Intent-to-Treat Analysis Set

	MMB (N=215)	RUX (N=217)	Total (N=432)
Stratum 6			
Transfusion Dependence at Baseline = 'No' and Platelet Count > 200*10E9/L			
RBC transfusion Rate in DB phase (units/month)			
N	109	110	219
Mean (SD)	0.1 (0.45)	0.5 (0.93)	0.3 (0.76)
Median	0.0	0.0	0.0
Q1, Q3	0.0, 0.0	0.0, 0.7	0.0, 0.2
Min, Max	0.0, 3.0	0.0, 4.3	0.0, 4.3
Negative Binomial Model			
Rate of RBC transfusion with 95% CI (units/month)	0.10 (0.05, 0.18)	0.55 (0.33, 0.92)	
Ratio of Rate for RBC Transfusion with 95% CI	0.18 (0.08, 0.39)	-	-
Proportional Means Model - Supportive Analysis			
Ratio of Mean Cumulative Function for RBC Transfusion with 95% CI	0.16 (0.07, 0.40)	-	-
Total number of RBC transfusion in stratum 6 in DB phase			
N	109	110	219
Mean (SD)	0.5 (2.03)	3.0 (5.15)	1.8 (4.12)
Median	0.0	0.0	0.0
Q1, Q3	0.0, 0.0	0.0, 4.0	0.0, 1.0
Min, Max	0.0, 14.0	0.0, 24.0	0.0, 24.0

Proportional means model is Andersen-Gill model with robust sandwich variance estimate.

RBC = Red Blood Cell

Source: Listing 16.3.3.1.1

Data Extracted: CRF data: 01JUL2019

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Table 15.12.3.1: Analysis of Rate of RBC Transfusion
Double-Blind Phase
Intent-to-Treat Analysis Set

	MMB (N=215)	RUX (N=217)	Total (N=432)
Duration of the DB phase (months) in stratum 6			
N	109	110	219
Mean (SD)	5.12 (1.269)	5.53 (0.409)	5.33 (0.961)
Median	5.55	5.55	5.55
Q1, Q3	5.45, 5.59	5.52, 5.62	5.52, 5.59
Min, Max	0.03, 5.78	1.38, 5.78	0.03, 5.78

Proportional means model is Andersen-Gill model with robust sandwich variance estimate.

RBC = Red Blood Cell

Source: Listing 16.3.3.1.1

Data Extracted: CRF data: 01JUL2019

Source: ...\\version5\\prog\\t-rbc24.sas v9.4 Output file: t-rbc24.out 09MAR2021:17:32