

Table 15.12.4.1: Analysis of RBC Transfusion Independent Response Rate at Week 24 and at Week 12
Double-Blind Phase
Intent-to-Treat Analysis Set

	MMB (N=215)	RUX (N=217)	Total (N=432)
All Strata Combined			
RBC Transfusion Independent Rate at Week 24			
Responder, n(%)	143 (66.5%)	107 (49.3%)	250 (57.9%)
95% Exact CI	0.5978, 0.7279	0.4248, 0.5616	0.5306, 0.6257
Proportion Difference - Stratified CMH Method (95% CI)	0.18 (0.09, 0.26)		
p-value	<0.001		
Proportion Difference - Unstratified Method (95% CI)	0.17 (0.08, 0.26)		
p-value	<0.001		
Proportion Difference - Unstratified Exact Method (95% CI)	0.17 (0.08, 0.27)		
p-value	<0.001		
Non-Responder, n(%)	72 (33.5%)	110 (50.7%)	182 (42.1%)
Transfusion(except bleeding) in the last 12 weeks	32 (14.9%)	87 (40.1%)	119 (27.5%)
Any Hgb assessment < 8g/dL in the last 12 weeks	27 (12.6%)	68 (31.3%)	95 (22.0%)
Last Participation date < Day 162 in DB phase	32 (14.9%)	12 (5.5%)	44 (10.2%)
Other	17 (7.9%)	34 (15.7%)	51 (11.8%)

95% Exact CI is based on Clopper-Pearson method without stratification.

CMH = Cochran-Mantel-Haenszel

RBC = Red Blood Cell

Source: Listing 16.3.3.2

Data Extracted: CRF data: 01JUL2019

Source: ...version5\prog\t-tf-ti.sas v9.4 Output file: t-rbcti24.out 09MAR2021:19:31

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RBC Transfusion Independent Rate at Week 12			
Responder, n(%)	147 (68.4%)	102 (47.0%)	249 (57.6%)
95% Exact CI	0.6170, 0.7453	0.4021, 0.5388	0.5282, 0.6235
Proportion Difference - Stratified CMH Method (95% CI)	0.22 (0.14, 0.30)		
p-value	<0.001		
Proportion Difference - Unstratified Method (95% CI)	0.21 (0.12, 0.30)		
p-value	<0.001		
Proportion Difference - Unstratified Exact Method (95% CI)	0.21 (0.12, 0.31)		
p-value	<0.001		
Non-Responder, n(%)	68 (31.6%)	115 (53.0%)	183 (42.4%)
Transfusion(except bleeding) in the last 12 weeks	45 (20.9%)	98 (45.2%)	143 (33.1%)
Any Hgb assessment < 8g/dL in the last 12 weeks	26 (12.1%)	92 (42.4%)	118 (27.3%)
Last Participation date < Day 78 in DB phase	16 (7.4%)	2 (0.9%)	18 (4.2%)
Other	17 (7.9%)	26 (12.0%)	43 (10.0%)

95% Exact CI is based on Clopper-Pearson method without stratification.

CMH = Cochran-Mantel-Haenszel

RBC = Red Blood Cell

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	MMB (N=215)	RUX (N=217)	Total (N=432)
Stratum 1			
Transfusion Dependence Yes and Platelet Count < 100X10E9/L	10	11	21
RBC Transfusion Independent Rate at Week 24			
Responder, n(%)	5 (50.0%)	2 (18.2%)	7 (33.3%)
95% Exact CI	0.1871, 0.8129	0.0228, 0.5178	0.1459, 0.5697
Proportion Difference (95% CI)	0.32 (-0.09, 0.73)		
Proportion Difference using Exact Method (95% CI)	0.32 (-0.14, 0.68)		
Non-Responder, n(%)	5 (50.0%)	9 (81.8%)	14 (66.7%)
Transfusion(except bleeding) in the last 12 weeks	4 (40.0%)	6 (54.5%)	10 (47.6%)
Any Hgb assessment < 8g/dL in the last 12 weeks	3 (30.0%)	5 (45.5%)	8 (38.1%)
Last Participation date < Day 162 in DB phase	1 (10.0%)	3 (27.3%)	4 (19.0%)
Other	1 (10.0%)	3 (27.3%)	4 (19.0%)

95% Exact CI is based on Clopper-Pearson method without stratification.

CMH = Cochran-Mantel-Haenszel

RBC = Red Blood Cell

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RBC Transfusion Independent Rate at Week 12			
Responder, n(%)	2 (20.0%)	1 (9.1%)	3 (14.3%)
95% Exact CI	0.0252, 0.5561	0.0023, 0.4128	0.0305, 0.3634
Proportion Difference (95% CI)	0.11 (-0.23, 0.45)		
Proportion Difference using Exact Method (95% CI)	0.11 (-0.32, 0.49)		
Non-Responder, n(%)	8 (80.0%)	10 (90.9%)	18 (85.7%)
Transfusion(except bleeding) in the last 12 weeks	7 (70.0%)	9 (81.8%)	16 (76.2%)
Any Hgb assessment < 8g/dL in the last 12 weeks	4 (40.0%)	8 (72.7%)	12 (57.1%)
Last Participation date < Day 78 in DB phase	1 (10.0%)	0	1 (4.8%)
Other	1 (10.0%)	4 (36.4%)	5 (23.8%)

95% Exact CI is based on Clopper-Pearson method without stratification.

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	MMB (N=215)	RUX (N=217)	Total (N=432)
Stratum 2			
Transfusion Dependence Yes and Platelet Count $\geq 100 \times 10^9/L$ and $\leq 200 \times 10^9/L$	21	20	41
RBC Transfusion Independent Rate at Week 24			
Responder, n(%)	7 (33.3%)	4 (20.0%)	11 (26.8%)
95% Exact CI	0.1459, 0.5697	0.0573, 0.4366	0.1422, 0.4294
Proportion Difference (95% CI)	0.13 (-0.14, 0.41)		
Proportion Difference using Exact Method (95% CI)	0.13 (-0.16, 0.43)		
Non-Responder, n(%)	14 (66.7%)	16 (80.0%)	30 (73.2%)
Transfusion(except bleeding) in the last 12 weeks	8 (38.1%)	8 (40.0%)	16 (39.0%)
Any Hgb assessment $< 8g/dL$ in the last 12 weeks	7 (33.3%)	8 (40.0%)	15 (36.6%)
Last Participation date $<$ Day 162 in DB phase	5 (23.8%)	5 (25.0%)	10 (24.4%)
Other	4 (19.0%)	5 (25.0%)	9 (22.0%)

95% Exact CI is based on Clopper-Pearson method without stratification.

CMH = Cochran-Mantel-Haenszel

RBC = Red Blood Cell

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RBC Transfusion Independent Rate at Week 12			
Responder, n(%)	4 (19.0%)	2 (10.0%)	6 (14.6%)
95% Exact CI	0.0545, 0.4191	0.0123, 0.3170	0.0557, 0.2917
Proportion Difference (95% CI)	0.09 (-0.14, 0.32)		
Proportion Difference using Exact Method (95% CI)	0.09 (-0.21, 0.39)		
Non-Responder, n(%)	17 (81.0%)	18 (90.0%)	35 (85.4%)
Transfusion(except bleeding) in the last 12 weeks	13 (61.9%)	15 (75.0%)	28 (68.3%)
Any Hgb assessment < 8g/dL in the last 12 weeks	10 (47.6%)	15 (75.0%)	25 (61.0%)
Last Participation date < Day 78 in DB phase	3 (14.3%)	1 (5.0%)	4 (9.8%)
Other	6 (28.6%)	9 (45.0%)	15 (36.6%)

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Stratum 3			
Transfusion Dependence Yes and Platelet Count > 200X10E9/L	22	21	43
RBC Transfusion Independent Rate at Week 24			
Responder, n(%)	4 (18.2%)	3 (14.3%)	7 (16.3%)
95% Exact CI	0.0519, 0.4028	0.0305, 0.3634	0.0681, 0.3070
Proportion Difference (95% CI)	0.04 (-0.19, 0.27)		
Proportion Difference using Exact Method (95% CI)	0.04 (-0.25, 0.33)		
Non-Responder, n(%)	18 (81.8%)	18 (85.7%)	36 (83.7%)
Transfusion(except bleeding) in the last 12 weeks	11 (50.0%)	17 (81.0%)	28 (65.1%)
Any Hgb assessment < 8g/dL in the last 12 weeks	11 (50.0%)	15 (71.4%)	26 (60.5%)
Last Participation date < Day 162 in DB phase	4 (18.2%)	0	4 (9.3%)
Other	7 (31.8%)	8 (38.1%)	15 (34.9%)

95% Exact CI is based on Clopper-Pearson method without stratification.

CMH = Cochran-Mantel-Haenszel

RBC = Red Blood Cell

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RBC Transfusion Independent Rate at Week 12			
Responder, n(%)	4 (18.2%)	2 (9.5%)	6 (14.0%)
95% Exact CI	0.0519, 0.4028	0.0117, 0.3038	0.0530, 0.2793
Proportion Difference (95% CI)	0.09 (-0.13, 0.31)		
Proportion Difference using Exact Method (95% CI)	0.09 (-0.20, 0.38)		
Non-Responder, n(%)	18 (81.8%)	19 (90.5%)	37 (86.0%)
Transfusion(except bleeding) in the last 12 weeks	13 (59.1%)	18 (85.7%)	31 (72.1%)
Any Hgb assessment < 8g/dL in the last 12 weeks	9 (40.9%)	16 (76.2%)	25 (58.1%)
Last Participation date < Day 78 in DB phase	3 (13.6%)	0	3 (7.0%)
Other	6 (27.3%)	7 (33.3%)	13 (30.2%)

95% Exact CI is based on Clopper-Pearson method without stratification.

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Stratum 4			
Transfusion Dependence No and Platelet Count < 100X10E9/L	8	12	20
RBC Transfusion Independent Rate at Week 24			
Responder, n(%)	6 (75.0%)	7 (58.3%)	13 (65.0%)
95% Exact CI	0.3491, 0.9681	0.2767, 0.8483	0.4078, 0.8461
Proportion Difference (95% CI)	0.17 (-0.28, 0.61)		
Proportion Difference using Exact Method (95% CI)	0.17 (-0.29, 0.58)		
Non-Responder, n(%)	2 (25.0%)	5 (41.7%)	7 (35.0%)
Transfusion(except bleeding) in the last 12 weeks	1 (12.5%)	3 (25.0%)	4 (20.0%)
Any Hgb assessment < 8g/dL in the last 12 weeks	0	2 (16.7%)	2 (10.0%)
Last Participation date < Day 162 in DB phase	1 (12.5%)	1 (8.3%)	2 (10.0%)
Other	0	0	0

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RBC Transfusion Independent Rate at Week 12			
Responder, n(%)	6 (75.0%)	7 (58.3%)	13 (65.0%)
95% Exact CI	0.3491, 0.9681	0.2767, 0.8483	0.4078, 0.8461
Proportion Difference (95% CI)	0.17 (-0.28, 0.61)		
Proportion Difference using Exact Method (95% CI)	0.17 (-0.29, 0.58)		
Non-Responder, n(%)	2 (25.0%)	5 (41.7%)	7 (35.0%)
Transfusion(except bleeding) in the last 12 weeks	1 (12.5%)	3 (25.0%)	4 (20.0%)
Any Hgb assessment < 8g/dL in the last 12 weeks	1 (12.5%)	3 (25.0%)	4 (20.0%)
Last Participation date < Day 78 in DB phase	1 (12.5%)	0	1 (5.0%)
Other	0	1 (8.3%)	1 (5.0%)

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	MMB (N=215)	RUX (N=217)	Total (N=432)
Stratum 5			
Transfusion Dependence No and Platelet Count $\geq 100 \times 10^9/L$ and $\leq 200 \times 10^9/L$	45	43	88
RBC Transfusion Independent Rate at Week 24			
Responder, n(%)	36 (80.0%)	24 (55.8%)	60 (68.2%)
95% Exact CI	0.6540, 0.9042	0.3988, 0.7092	0.5739, 0.7771
Proportion Difference (95% CI)	0.24 (0.05, 0.43)		
Proportion Difference using Exact Method (95% CI)	0.24 (0.03, 0.43)		
Non-Responder, n(%)	9 (20.0%)	19 (44.2%)	28 (31.8%)
Transfusion(except bleeding) in the last 12 weeks	3 (6.7%)	16 (37.2%)	19 (21.6%)
Any Hgb assessment $< 8g/dL$ in the last 12 weeks	3 (6.7%)	9 (20.9%)	12 (13.6%)
Last Participation date $<$ Day 162 in DB phase	4 (8.9%)	2 (4.7%)	6 (6.8%)
Other	2 (4.4%)	5 (11.6%)	7 (8.0%)

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RBC Transfusion Independent Rate at Week 12			
Responder, n(%)	40 (88.9%)	22 (51.2%)	62 (70.5%)
95% Exact CI	0.7595, 0.9629	0.3546, 0.6669	0.5978, 0.7971
Proportion Difference (95% CI)	0.38 (0.20, 0.56)		
Proportion Difference using Exact Method (95% CI)	0.38 (0.17, 0.56)		
Non-Responder, n(%)	5 (11.1%)	21 (48.8%)	26 (29.5%)
Transfusion(except bleeding) in the last 12 weeks	2 (4.4%)	18 (41.9%)	20 (22.7%)
Any Hgb assessment < 8g/dL in the last 12 weeks	0	17 (39.5%)	17 (19.3%)
Last Participation date < Day 78 in DB phase	1 (2.2%)	0	1 (1.1%)
Other	2 (4.4%)	2 (4.7%)	4 (4.5%)

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	MMB (N=215)	RUX (N=217)	Total (N=432)
Stratum 6			
Transfusion Dependence No and Platelet Count > 200X10E9/L	109	110	219
RBC Transfusion Independent Rate at Week 24			
Responder, n(%)	85 (78.0%)	67 (60.9%)	152 (69.4%)
95% Exact CI	0.6903, 0.8535	0.5114, 0.7007	0.6284, 0.7544
Proportion Difference (95% CI)	0.17 (0.05, 0.29)		
Proportion Difference using Exact Method (95% CI)	0.17 (0.04, 0.30)		
Non-Responder, n(%)	24 (22.0%)	43 (39.1%)	67 (30.6%)
Transfusion(except bleeding) in the last 12 weeks	5 (4.6%)	37 (33.6%)	42 (19.2%)
Any Hgb assessment < 8g/dL in the last 12 weeks	3 (2.8%)	29 (26.4%)	32 (14.6%)
Last Participation date < Day 162 in DB phase	17 (15.6%)	1 (0.9%)	18 (8.2%)
Other	3 (2.8%)	13 (11.8%)	16 (7.3%)

95% Exact CI is based on Clopper-Pearson method without stratification.

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RBC Transfusion Independent Rate at Week 12			
Responder, n(%)	91 (83.5%)	68 (61.8%)	159 (72.6%)
95% Exact CI	0.7516, 0.8991	0.5207, 0.7092	0.6619, 0.7840
Proportion Difference (95% CI)	0.22 (0.10, 0.33)		
Proportion Difference using Exact Method (95% CI)	0.22 (0.09, 0.34)		
Non-Responder, n(%)	18 (16.5%)	42 (38.2%)	60 (27.4%)
Transfusion(except bleeding) in the last 12 weeks	9 (8.3%)	35 (31.8%)	44 (20.1%)
Any Hgb assessment < 8g/dL in the last 12 weeks	2 (1.8%)	33 (30.0%)	35 (16.0%)
Last Participation date < Day 78 in DB phase	7 (6.4%)	1 (0.9%)	8 (3.7%)
Other	2 (1.8%)	3 (2.7%)	5 (2.3%)

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