

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

	MMB (N=104)	BAT (N=52)	Total (N=156)
All Strata Combined			
RBC Transfusion Independent Rate at Week 24			
Responder, n(%)	45 (43.3%)	11 (21.2%)	56 (35.9%)
95% Exact CI	0.3359, 0.5335	0.1106, 0.3470	0.2838, 0.4396
Proportion Difference - Stratified CMH Method (95% CI)	0.23 (0.09, 0.37)		
p-value	0.001		
Proportion Difference - Unstratified Method (95% CI)	0.22 (0.07, 0.37)		
p-value	0.003		
Proportion Difference - Unstratified Exact Method (95% CI)	0.22 (0.05, 0.38)		
p-value	0.008		
Non-Responder, n(%)	59 (56.7%)	41 (78.8%)	100 (64.1%)
Transfusion (except bleeding) in the last 12 weeks	30 (28.8%)	28 (53.8%)	58 (37.2%)
Any Hgb assessment < 8g/dL in the last 12 weeks	23 (22.1%)	20 (38.5%)	43 (27.6%)
Last Participation date < Day 162 in RT phase	28 (26.9%)	10 (19.2%)	38 (24.4%)
Other	11 (10.6%)	4 (7.7%)	15 (9.6%)

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: 25JUN2019

Source: ...\\version4\prog\t-tf-ti.sas v9.4 Output file: t-rbcti24.out 27AUG2020:21:00

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RBC Transfusion Independent Rate at Week 12			
Responder, n(%)	40 (38.5%)	9 (17.3%)	49 (31.4%)
95% Exact CI	0.2909, 0.4851	0.0823, 0.3033	0.2422, 0.3932
Proportion Difference - Stratified CMH Method (95% CI)	0.23 (0.10, 0.36)		
p-value	<0.001		
Proportion Difference - Unstratified Method (95% CI)	0.21 (0.07, 0.35)		
p-value	0.003		
Proportion Difference - Unstratified Exact Method (95% CI)	0.21 (0.04, 0.37)		
p-value	0.010		
Non-Responder, n(%)	64 (61.5%)	43 (82.7%)	107 (68.6%)
Transfusion (except bleeding) in the last 12 weeks	46 (44.2%)	32 (61.5%)	78 (50.0%)
Any Hgb assessment < 8g/dL in the last 12 weeks	37 (35.6%)	22 (42.3%)	59 (37.8%)
Last Participation date < Day 78 in RT phase	14 (13.5%)	9 (17.3%)	23 (14.7%)
Other	23 (22.1%)	6 (11.5%)	29 (18.6%)

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

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Stratum 1			
Transfusion Dependence Yes and Baseline TSS <18	37	15	52
RBC Transfusion Independent Rate at Week 24			
Responder, n(%)	14 (37.8%)	0	14 (26.9%)
95% Exact CI	0.2246, 0.5524	0.0000, 0.2180	0.1557, 0.4102
Proportion Difference (95% CI)	0.38 (0.20, 0.56)		
Proportion Difference using Exact Method (95% CI)	0.38 (0.08, 0.64)		
Non-Responder, n(%)	23 (62.2%)	15 (100.0%)	38 (73.1%)
Transfusion (except bleeding) in the last 12 weeks	16 (43.2%)	11 (73.3%)	27 (51.9%)
Any Hgb assessment < 8g/dL in the last 12 weeks	12 (32.4%)	6 (40.0%)	18 (34.6%)
Last Participation date < Day 162 in RT phase	7 (18.9%)	3 (20.0%)	10 (19.2%)
Other	7 (18.9%)	2 (13.3%)	9 (17.3%)

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

CMH = Cochran-Mantel-Haenszel;

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RBC Transfusion Independent Rate at Week 12			
Responder, n(%)	7 (18.9%)	0	7 (13.5%)
95% Exact CI	0.0796, 0.3516	0.0000, 0.2180	0.0559, 0.2579
Proportion Difference (95% CI)	0.19 (0.03, 0.35)		
Proportion Difference using Exact Method (95% CI)	0.19 (-0.11, 0.47)		
Non-Responder, n(%)	30 (81.1%)	15 (100.0%)	45 (86.5%)
Transfusion (except bleeding) in the last 12 weeks	26 (70.3%)	12 (80.0%)	38 (73.1%)
Any Hgb assessment < 8g/dL in the last 12 weeks	22 (59.5%)	9 (60.0%)	31 (59.6%)
Last Participation date < Day 78 in RT phase	1 (2.7%)	3 (20.0%)	4 (7.7%)
Other	14 (37.8%)	3 (20.0%)	17 (32.7%)

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

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Stratum 2			
Transfusion Dependence Yes and Baseline TSS >=18	21	12	33
RBC Transfusion Independent Rate at Week 24			
Responder, n(%)	5 (23.8%)	1 (8.3%)	6 (18.2%)
95% Exact CI	0.0822, 0.4717	0.0021, 0.3848	0.0698, 0.3546
Proportion Difference (95% CI)	0.15 (-0.11, 0.42)		
Proportion Difference using Exact Method (95% CI)	0.15 (-0.21, 0.49)		
Non-Responder, n(%)	16 (76.2%)	11 (91.7%)	27 (81.8%)
Transfusion (except bleeding) in the last 12 weeks	8 (38.1%)	8 (66.7%)	16 (48.5%)
Any Hgb assessment < 8g/dL in the last 12 weeks	7 (33.3%)	7 (58.3%)	14 (42.4%)
Last Participation date < Day 162 in RT phase	8 (38.1%)	3 (25.0%)	11 (33.3%)
Other	2 (9.5%)	1 (8.3%)	3 (9.1%)

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

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RBC Transfusion Independent Rate at Week 12			
Responder, n(%)	5 (23.8%)	0	5 (15.2%)
95% Exact CI	0.0822, 0.4717	0.0000, 0.2646	0.0511, 0.3190
Proportion Difference (95% CI)	0.24 (0.02, 0.46)		
Proportion Difference using Exact Method (95% CI)	0.24 (-0.13, 0.56)		
Non-Responder, n(%)	16 (76.2%)	12 (100.0%)	28 (84.8%)
Transfusion (except bleeding) in the last 12 weeks	11 (52.4%)	9 (75.0%)	20 (60.6%)
Any Hgb assessment < 8g/dL in the last 12 weeks	11 (52.4%)	8 (66.7%)	19 (57.6%)
Last Participation date < Day 78 in RT phase	5 (23.8%)	3 (25.0%)	8 (24.2%)
Other	7 (33.3%)	1 (8.3%)	8 (24.2%)

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

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Stratum 3			
Transfusion Dependence No and Baseline TSS <18	24	13	37
RBC Transfusion Independent Rate at Week 24			
Responder, n(%)	18 (75.0%)	7 (53.8%)	25 (67.6%)
95% Exact CI	0.5329, 0.9023	0.2513, 0.8078	0.5021, 0.8199
Proportion Difference (95% CI)	0.21 (-0.12, 0.55)		
Proportion Difference using Exact Method (95% CI)	0.21 (-0.12, 0.53)		
Non-Responder, n(%)	6 (25.0%)	6 (46.2%)	12 (32.4%)
Transfusion (except bleeding) in the last 12 weeks	1 (4.2%)	4 (30.8%)	5 (13.5%)
Any Hgb assessment < 8g/dL in the last 12 weeks	1 (4.2%)	5 (38.5%)	6 (16.2%)
Last Participation date < Day 162 in RT phase	5 (20.8%)	1 (7.7%)	6 (16.2%)
Other	1 (4.2%)	0	1 (2.7%)

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RBC Transfusion Independent Rate at Week 12			
Responder, n(%)	16 (66.7%)	6 (46.2%)	22 (59.5%)
95% Exact CI	0.4468, 0.8437	0.1922, 0.7487	0.4210, 0.7525
Proportion Difference (95% CI)	0.21 (-0.14, 0.55)		
Proportion Difference using Exact Method (95% CI)	0.21 (-0.13, 0.52)		
Non-Responder, n(%)	8 (33.3%)	7 (53.8%)	15 (40.5%)
Transfusion (except bleeding) in the last 12 weeks	4 (16.7%)	5 (38.5%)	9 (24.3%)
Any Hgb assessment < 8g/dL in the last 12 weeks	2 (8.3%)	4 (30.8%)	6 (16.2%)
Last Participation date < Day 78 in RT phase	3 (12.5%)	0	3 (8.1%)
Other	2 (8.3%)	2 (15.4%)	4 (10.8%)

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Stratum 4			
Transfusion Dependence No and Baseline TSS >=18	22	12	34
RBC Transfusion Independent Rate at Week 24			
Responder, n(%)	8 (36.4%)	3 (25.0%)	11 (32.4%)
95% Exact CI	0.1720, 0.5934	0.0549, 0.5719	0.1739, 0.5053
Proportion Difference (95% CI)	0.11 (-0.22, 0.45)		
Proportion Difference using Exact Method (95% CI)	0.11 (-0.24, 0.45)		
Non-Responder, n(%)	14 (63.6%)	9 (75.0%)	23 (67.6%)
Transfusion (except bleeding) in the last 12 weeks	5 (22.7%)	5 (41.7%)	10 (29.4%)
Any Hgb assessment < 8g/dL in the last 12 weeks	3 (13.6%)	2 (16.7%)	5 (14.7%)
Last Participation date < Day 162 in RT phase	8 (36.4%)	3 (25.0%)	11 (32.4%)
Other	1 (4.5%)	1 (8.3%)	2 (5.9%)

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RBC Transfusion Independent Rate at Week 12			
Responder, n(%)	12 (54.5%)	3 (25.0%)	15 (44.1%)
95% Exact CI	0.3221, 0.7561	0.0549, 0.5719	0.2719, 0.6211
Proportion Difference (95% CI)	0.30 (-0.04, 0.63)		
Proportion Difference using Exact Method (95% CI)	0.30 (-0.07, 0.60)		
Non-Responder, n(%)	10 (45.5%)	9 (75.0%)	19 (55.9%)
Transfusion (except bleeding) in the last 12 weeks	5 (22.7%)	6 (50.0%)	11 (32.4%)
Any Hgb assessment < 8g/dL in the last 12 weeks	2 (9.1%)	1 (8.3%)	3 (8.8%)
Last Participation date < Day 78 in RT phase	5 (22.7%)	3 (25.0%)	8 (23.5%)
Other	0	0	0

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