

Table 15.12.2.1: Analysis of Response Rate in TSS 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			
Total Symptom Score Status			
Missing TSS at baseline (excluded from rate calculation)	2 ( 0.9%)	3 ( 1.4%)	5 ( 1.2%)
TSS = 0 at baseline	3 ( 1.4%)	3 ( 1.4%)	6 ( 1.4%)
TSS > 0 at baseline	210 ( 97.7%)	211 ( 97.2%)	421 ( 97.5%)
Response Rate of Total Symptom Score at Week 24			
Subjects Evaluable at Week 24, n	211	211	422
TSS = 0 at baseline and TSS >0 or missing at Week 24	1 ( 0.5%)	0	1 ( 0.2%)
Responder, n(%)			
95% Exact CI	60 ( 28.4%)	89 ( 42.2%)	149 ( 35.3%)
Noninferior Proportion Difference - Stratified CMH Method (95% CI)	0.2245, 0.3503	0.3543, 0.4915	0.3075, 0.4008
p-value	0.00 (-0.08, 0.08)		
Noninferior Proportion Difference - Unstratified CMH Method (95% CI)	0.98		
p-value	0.00 (-0.07, 0.08)		
Superior Proportion Difference - Stratified CMH Method (95% CI)	0.96		
p-value	-0.14 (-0.23, -0.05)		
Superior Proportion Difference - Unstratified CMH Method (95% CI)	0.003		
p-value	-0.14 (-0.23, -0.05)		
Superior Proportion Difference - Unstratified Exact Method (95% CI)	0.003		
p-value	-0.14 (-0.23, -0.04)		
Non-Responder, n(%)			
Last participation date < Day 162 in DB phase	0.004		
Last participation date >= Day 162 and TSS at Week 24 not available	151 ( 71.6%)	122 ( 57.8%)	273 ( 64.7%)
>0% increase from baseline at Week 24	31 ( 14.7%)	12 ( 5.7%)	43 ( 10.2%)
<50% reduction from baseline week 24	5 ( 2.4%)	9 ( 4.3%)	14 ( 3.3%)
	47 ( 22.3%)	32 ( 15.2%)	79 ( 18.7%)
	114 ( 54.0%)	101 ( 47.9%)	215 ( 50.9%)

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;

Source: Listing 16.3.2.1

Data Extracted: CRF data: 01JUL2019

Source: ...\\version5\\prog\\t-tss24.sas v9.4 Output file: t-tss24.out 09MAR2021:19:52

Table 15.12.2.1: Analysis of Response Rate in TSS at Week 24 and at Week 12  
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Intent-to-Treat Analysis Set

	MMB (N=215)	RUX (N=217)	Total (N=432)
<b>Response Rate of Total Symptom Score at Week 12</b>			
Subjects Evaluable at Week 12, n	212	213	425
TSS = 0 at baseline and TSS >0 or missing at Week 12	2 ( 0.9%)	2 ( 0.9%)	4 ( 0.9%)
<b>Responder, n(%)</b>	<b>58 ( 27.4%)</b>	<b>74 ( 34.7%)</b>	<b>132 ( 31.1%)</b>
95% Exact CI	0.2148, 0.3389	0.2837, 0.4155	0.2669, 0.3570
Noninferior Proportion Difference - Stratified CMH Method (95% CI)	0.04 (-0.03, 0.12)		
p-value	0.28		
Noninferior Proportion Difference - Unstratified CMH Method (95% CI)	0.04 (-0.03, 0.11)		
p-value	0.28		
Superior Proportion Difference - Stratified CMH Method (95% CI)	-0.07 (-0.16, 0.02)		
p-value	0.11		
Superior Proportion Difference - Unstratified CMH Method (95% CI)	-0.07 (-0.16, 0.01)		
p-value	0.100		
Superior Proportion Difference - Unstratified Exact Method (95% CI)	-0.07 (-0.17, 0.02)		
p-value	0.12		
<b>Non-Responder, n(%)</b>	<b>154 ( 72.6%)</b>	<b>139 ( 65.3%)</b>	<b>293 ( 68.9%)</b>
Last participation date < Day 78 in DB phase	16 ( 7.5%)	2 ( 0.9%)	18 ( 4.2%)
Last participation date >= Day 78 and TSS at Week 12 not available	5 ( 2.4%)	7 ( 3.3%)	12 ( 2.8%)
>0% increase from baseline at Week 12	50 ( 23.6%)	37 ( 17.4%)	87 ( 20.5%)
<50% reduction from baseline at week 12	131 ( 61.8%)	128 ( 60.1%)	259 ( 60.9%)

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TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

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Double-Blind Phase  
Intent-to-Treat Analysis Set

	MMB (N=215)	RUX (N=217)	Total (N=432)
Stratum 1			
Transfusion Dependence Yes and Platelet Count < 100X10E9/L	10	11	21
Total Symptom Score Status			
Missing TSS at baseline (excluded from rate calculation)	1 ( 10.0%)	0	1 ( 4.8%)
TSS = 0 at baseline	0	0	0
TSS > 0 at baseline	9 ( 90.0%)	11 (100.0%)	20 ( 95.2%)
Response Rate of Total Symptom Score at Week 24			
Subjects Evaluable at Week 24, n	9	11	20
TSS = 0 at baseline and TSS >0 or missing at Week 24	0	0	0
Responder, n(%)	2 ( 22.2%)	3 ( 27.3%)	5 ( 25.0%)
95% Exact CI	0.0281, 0.6001	0.0602, 0.6097	0.0866, 0.4910
Noninferior Proportion Difference - Unstratified CMH Method (95% CI)	0.04 (-0.31, 0.39)		
Superior Proportion Difference - Unstratified CMH Method (95% CI)	-0.05 (-0.46, 0.36)		
Superior Proportion Difference - Unstratified Exact Method (95% CI)	-0.05 (-0.46, 0.38)		
Non-Responder, n(%)	7 ( 77.8%)	8 ( 72.7%)	15 ( 75.0%)
Last participation date < Day 162 in DB phase	1 ( 11.1%)	3 ( 27.3%)	4 ( 20.0%)
Last participation date >= Day 162 and TSS at Week 24 not available	0	1 ( 9.1%)	1 ( 5.0%)
>0% increase from baseline at Week 24	4 ( 44.4%)	1 ( 9.1%)	5 ( 25.0%)
<50% reduction from baseline week 24	6 ( 66.7%)	4 ( 36.4%)	10 ( 50.0%)

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;

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	MMB (N=215)	RUX (N=217)	Total (N=432)
<b>Response Rate of Total Symptom Score at Week 12</b>			
Subjects Evaluable at Week 12, n	9	11	20
TSS = 0 at baseline and TSS >0 or missing at Week 12	0	0	0
<b>Responder, n(%)</b>	<b>2 ( 22.2%)</b>	<b>4 ( 36.4%)</b>	<b>6 ( 30.0%)</b>
95% Exact CI	0.0281, 0.6001	0.1093, 0.6921	0.1189, 0.5428
Noninferior Proportion Difference - Unstratified CMH Method (95% CI)	-0.02 (-0.38, 0.34)		
Superior Proportion Difference - Unstratified CMH Method (95% CI)	-0.14 (-0.57, 0.28)		
Superior Proportion Difference - Unstratified Exact Method (95% CI)	-0.14 (-0.54, 0.29)		
<b>Non-Responder, n(%)</b>	<b>7 ( 77.8%)</b>	<b>7 ( 63.6%)</b>	<b>14 ( 70.0%)</b>
Last participation date < Day 78 in DB phase	1 ( 11.1%)	0	1 ( 5.0%)
Last participation date >= Day 78 and TSS at Week 12 not available	0	1 ( 9.1%)	1 ( 5.0%)
>0% increase from baseline at Week 12	5 ( 55.6%)	2 ( 18.2%)	7 ( 35.0%)
<50% reduction from baseline at week 12	6 ( 66.7%)	6 ( 54.5%)	12 ( 60.0%)

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TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

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Intent-to-Treat Analysis Set

	MMB (N=215)	RUX (N=217)	Total (N=432)
<b>Stratum 2</b>			
Transfusion Dependence Yes and Platelet Count $\geq 100 \times 10^9/L$ and $\leq 200 \times 10^9/L$	21	20	41
<b>Total Symptom Score Status</b>			
Missing TSS at baseline (excluded from rate calculation)	0	0	0
TSS = 0 at baseline	1 ( 4.8%)	0	1 ( 2.4%)
TSS > 0 at baseline	20 ( 95.2%)	20 (100.0%)	40 ( 97.6%)
<b>Response Rate of Total Symptom Score at Week 24</b>			
Subjects Evaluable at Week 24, n	20	20	40
TSS = 0 at baseline and TSS >0 or missing at Week 24	0	0	0
<b>Responder, n(%)</b>			
95% Exact CI	6 ( 30.0%)	8 ( 40.0%)	14 ( 35.0%)
Noninferior Proportion Difference - Unstratified CMH Method (95% CI)	0.1189, 0.5428	0.1912, 0.6395	0.2063, 0.5168
Superior Proportion Difference - Unstratified CMH Method (95% CI)	0.03 (-0.22, 0.29)		
Superior Proportion Difference - Unstratified Exact Method (95% CI)	-0.10 (-0.40, 0.20)		
Superior Proportion Difference - Unstratified Exact Method (95% CI)	-0.10 (-0.41, 0.23)		
<b>Non-Responder, n(%)</b>			
Last participation date < Day 162 in DB phase	14 ( 70.0%)	12 ( 60.0%)	26 ( 65.0%)
Last participation date $\geq$ Day 162 and TSS at Week 24 not available	5 ( 25.0%)	5 ( 25.0%)	10 ( 25.0%)
Last participation date $\geq$ Day 162 and TSS at Week 24 not available	1 ( 5.0%)	0	1 ( 2.5%)
>0% increase from baseline at Week 24	3 ( 15.0%)	3 ( 15.0%)	6 ( 15.0%)
<50% reduction from baseline week 24	8 ( 40.0%)	7 ( 35.0%)	15 ( 37.5%)

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;

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	MMB (N=215)	RUX (N=217)	Total (N=432)
<b>Response Rate of Total Symptom Score at Week 12</b>			
Subjects Evaluable at Week 12, n	20	20	40
TSS = 0 at baseline and TSS >0 or missing at Week 12	0	0	0
<b>Responder, n(%)</b>	<b>7 ( 35.0%)</b>	<b>7 ( 35.0%)</b>	<b>14 ( 35.0%)</b>
95% Exact CI	0.1539, 0.5922	0.1539, 0.5922	0.2063, 0.5168
Noninferior Proportion Difference - Unstratified CMH Method (95% CI)	0.12 (-0.14, 0.37)		
Superior Proportion Difference - Unstratified CMH Method (95% CI)	0.00 (-0.30, 0.30)		
Superior Proportion Difference - Unstratified Exact Method (95% CI)	0.00 (-0.32, 0.32)		
<b>Non-Responder, n(%)</b>	<b>13 ( 65.0%)</b>	<b>13 ( 65.0%)</b>	<b>26 ( 65.0%)</b>
Last participation date < Day 78 in DB phase	3 ( 15.0%)	1 ( 5.0%)	4 ( 10.0%)
Last participation date >= Day 78 and TSS at Week 12 not available	1 ( 5.0%)	0	1 ( 2.5%)
>0% increase from baseline at Week 12	2 ( 10.0%)	3 ( 15.0%)	5 ( 12.5%)
<50% reduction from baseline at week 12	9 ( 45.0%)	12 ( 60.0%)	21 ( 52.5%)

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TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

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	MMB (N=215)	RUX (N=217)	Total (N=432)
Stratum 3			
Transfusion Dependence Yes and Platelet Count > 200X10E9/L	22	21	43
Total Symptom Score Status			
Missing TSS at baseline (excluded from rate calculation)	0	2 ( 9.5%)	2 ( 4.7%)
TSS = 0 at baseline	0	0	0
TSS > 0 at baseline	22 (100.0%)	19 ( 90.5%)	41 ( 95.3%)
Response Rate of Total Symptom Score at Week 24			
Subjects Evaluable at Week 24, n	22	19	41
TSS = 0 at baseline and TSS >0 or missing at Week 24	0	0	0
Responder, n(%)	4 ( 18.2%)	9 ( 47.4%)	13 ( 31.7%)
95% Exact CI	0.0519, 0.4028	0.2445, 0.7114	0.1808, 0.4809
Noninferior Proportion Difference - Unstratified CMH Method (95% CI)	-0.14 (-0.37, 0.09)		
Superior Proportion Difference - Unstratified CMH Method (95% CI)	-0.29 (-0.58, -0.01)		
Superior Proportion Difference - Unstratified Exact Method (95% CI)	-0.29 (-0.56, 0.02)		
Non-Responder, n(%)	18 ( 81.8%)	10 ( 52.6%)	28 ( 68.3%)
Last participation date < Day 162 in DB phase	4 ( 18.2%)	0	4 ( 9.8%)
Last participation date >= Day 162 and TSS at Week 24 not available	0	2 ( 10.5%)	2 ( 4.9%)
>0% increase from baseline at Week 24	6 ( 27.3%)	1 ( 5.3%)	7 ( 17.1%)
<50% reduction from baseline week 24	14 ( 63.6%)	8 ( 42.1%)	22 ( 53.7%)

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;

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	MMB (N=215)	RUX (N=217)	Total (N=432)
<hr/>			
Response Rate of Total Symptom Score at Week 12			
Subjects Evaluable at Week 12, n	22	19	41
TSS = 0 at baseline and TSS >0 or missing at Week 12	0	0	0
Responder, n(%)	5 ( 22.7%)	6 ( 31.6%)	11 ( 26.8%)
95% Exact CI	0.0782, 0.4537	0.1258, 0.5655	0.1422, 0.4294
Noninferior Proportion Difference - Unstratified CMH Method (95% CI)	0.02 (-0.22, 0.25)		
Superior Proportion Difference - Unstratified CMH Method (95% CI)	-0.09 (-0.37, 0.19)		
Superior Proportion Difference - Unstratified Exact Method (95% CI)	-0.09 (-0.39, 0.21)		
Non-Responder, n(%)	17 ( 77.3%)	13 ( 68.4%)	30 ( 73.2%)
Last participation date < Day 78 in DB phase	3 ( 13.6%)	0	3 ( 7.3%)
Last participation date >= Day 78 and TSS at Week 12 not available	0	0	0
>0% increase from baseline at Week 12	6 ( 27.3%)	2 ( 10.5%)	8 ( 19.5%)
<50% reduction from baseline at week 12	14 ( 63.6%)	13 ( 68.4%)	27 ( 65.9%)

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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;

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	MMB (N=215)	RUX (N=217)	Total (N=432)
<b>Stratum 4</b>			
Transfusion Dependence No and Platelet Count < 100X10E9/L	8	12	20
<b>Total Symptom Score Status</b>			
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%)	12 (100.0%)	20 (100.0%)
<b>Response Rate of Total Symptom Score at Week 24</b>			
Subjects Evaluable at Week 24, n	8	12	20
TSS = 0 at baseline and TSS >0 or missing at Week 24	0	0	0
<b>Responder, n(%)</b>			
95% Exact CI	4 ( 50.0%)	2 ( 16.7%)	6 ( 30.0%)
Noninferior Proportion Difference - Unstratified CMH Method (95% CI)	0.1570, 0.8430	0.0209, 0.4841	0.1189, 0.5428
Superior Proportion Difference - Unstratified CMH Method (95% CI)	0.39 (-0.01, 0.79)		
Superior Proportion Difference - Unstratified Exact Method (95% CI)	0.33 (-0.10, 0.77)		
Superior Proportion Difference - Unstratified Exact Method (95% CI)	0.33 (-0.14, 0.71)		
<b>Non-Responder, n(%)</b>			
Last participation date < Day 162 in DB phase	4 ( 50.0%)	10 ( 83.3%)	14 ( 70.0%)
Last participation date >= Day 162 and TSS at Week 24 not available	1 ( 12.5%)	1 ( 8.3%)	2 ( 10.0%)
Last participation date >= Day 162 and TSS at Week 24 not available	0	0	0
>0% increase from baseline at Week 24	2 ( 25.0%)	2 ( 16.7%)	4 ( 20.0%)
<50% reduction from baseline week 24	3 ( 37.5%)	9 ( 75.0%)	12 ( 60.0%)

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TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

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	MMB (N=215)	RUX (N=217)	Total (N=432)
<b>Response Rate of Total Symptom Score at Week 12</b>			
Subjects Evaluable at Week 12, n	8	12	20
TSS = 0 at baseline and TSS >0 or missing at Week 12	0	0	0
<b>Responder, n(%)</b>	3 ( 37.5%)	3 ( 25.0%)	6 ( 30.0%)
95% Exact CI	0.0852, 0.7551	0.0549, 0.5719	0.1189, 0.5428
Noninferior Proportion Difference - Unstratified CMH Method (95% CI)	0.21 (-0.19, 0.61)		
Superior Proportion Difference - Unstratified CMH Method (95% CI)	0.13 (-0.32, 0.57)		
Superior Proportion Difference - Unstratified Exact Method (95% CI)	0.13 (-0.33, 0.55)		
<b>Non-Responder, n(%)</b>	5 ( 62.5%)	9 ( 75.0%)	14 ( 70.0%)
Last participation date < Day 78 in DB phase	1 ( 12.5%)	0	1 ( 5.0%)
Last participation date >= Day 78 and TSS at Week 12 not available	0	1 ( 8.3%)	1 ( 5.0%)
>0% increase from baseline at Week 12	2 ( 25.0%)	3 ( 25.0%)	5 ( 25.0%)
<50% reduction from baseline at week 12	4 ( 50.0%)	8 ( 66.7%)	12 ( 60.0%)

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<b>Stratum 5</b>			
Transfusion Dependence No and Platelet Count $\geq 100 \times 10^9/L$ and $\leq 200 \times 10^9/L$	45	43	88
<b>Total Symptom Score Status</b>			
Missing TSS at baseline (excluded from rate calculation)	0	0	0
TSS = 0 at baseline	1 ( 2.2%)	2 ( 4.7%)	3 ( 3.4%)
TSS > 0 at baseline	44 ( 97.8%)	41 ( 95.3%)	85 ( 96.6%)
<b>Response Rate of Total Symptom Score at Week 24</b>			
Subjects Evaluable at Week 24, n	44	41	85
TSS = 0 at baseline and TSS >0 or missing at Week 24	0	0	0
<b>Responder, n(%)</b>			
95% Exact CI	14 ( 31.8%)	17 ( 41.5%)	31 ( 36.5%)
Noninferior Proportion Difference - Unstratified CMH Method (95% CI)	0.1861, 0.4758	0.2632, 0.5789	0.2629, 0.4762
Superior Proportion Difference - Unstratified CMH Method (95% CI)	0.04 (-0.13, 0.21)		
Superior Proportion Difference - Unstratified Exact Method (95% CI)	-0.10 (-0.30, 0.11)		
Superior Proportion Difference - Unstratified Exact Method (95% CI)	-0.10 (-0.30, 0.12)		
<b>Non-Responder, n(%)</b>			
Last participation date < Day 162 in DB phase	30 ( 68.2%)	24 ( 58.5%)	54 ( 63.5%)
Last participation date $\geq$ Day 162 and TSS at Week 24 not available	4 ( 9.1%)	2 ( 4.9%)	6 ( 7.1%)
Last participation date $\geq$ Day 162 and TSS at Week 24 not available	2 ( 4.5%)	0	2 ( 2.4%)
>0% increase from baseline at Week 24	6 ( 13.6%)	7 ( 17.1%)	13 ( 15.3%)
<50% reduction from baseline week 24	24 ( 54.5%)	22 ( 53.7%)	46 ( 54.1%)

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TSS = Total Symptom Score; CMH = Cochran-Mantel-Haenszel;

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	MMB (N=215)	RUX (N=217)	Total (N=432)
<b>Response Rate of Total Symptom Score at Week 12</b>			
Subjects Evaluable at Week 12, n	45	43	88
TSS = 0 at baseline and TSS >0 or missing at Week 12	1 ( 2.2%)	2 ( 4.7%)	3 ( 3.4%)
<b>Responder, n(%)</b>	13 ( 28.9%)	11 ( 25.6%)	24 ( 27.3%)
95% Exact CI	0.1637, 0.4431	0.1352, 0.4117	0.1832, 0.3781
Noninferior Proportion Difference - Unstratified CMH Method (95% CI)	0.12 (-0.04, 0.28)		
Superior Proportion Difference - Unstratified CMH Method (95% CI)	0.03 (-0.16, 0.22)		
Superior Proportion Difference - Unstratified Exact Method (95% CI)	0.03 (-0.17, 0.24)		
<b>Non-Responder, n(%)</b>	32 ( 71.1%)	32 ( 74.4%)	64 ( 72.7%)
Last participation date < Day 78 in DB phase	1 ( 2.2%)	0	1 ( 1.1%)
Last participation date >= Day 78 and TSS at Week 12 not available	0	1 ( 2.3%)	1 ( 1.1%)
>0% increase from baseline at Week 12	11 ( 24.4%)	10 ( 23.3%)	21 ( 23.9%)
<50% reduction from baseline at week 12	30 ( 66.7%)	29 ( 67.4%)	59 ( 67.0%)

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;

Source: Listing 16.3.2.1

Data Extracted: CRF data: 01JUL2019

Source: ...\\version5\\prog\\t-tss24.sas v9.4 Output file: t-tss24.out 09MAR2021:19:52

Table 15.12.2.1: Analysis of Response Rate in TSS at Week 24 and at Week 12  
Double-Blind Phase  
Intent-to-Treat Analysis Set

	MMB (N=215)	RUX (N=217)	Total (N=432)
<hr/>			
Stratum 6			
Transfusion Dependence No and Platelet Count > 200X10E9/L	109	110	219
Total Symptom Score Status			
Missing TSS at baseline (excluded from rate calculation)	1 ( 0.9%)	1 ( 0.9%)	2 ( 0.9%)
TSS = 0 at baseline	1 ( 0.9%)	1 ( 0.9%)	2 ( 0.9%)
TSS > 0 at baseline	107 ( 98.2%)	108 ( 98.2%)	215 ( 98.2%)
Response Rate of Total Symptom Score at Week 24			
Subjects Evaluable at Week 24, n	108	108	216
TSS = 0 at baseline and TSS >0 or missing at Week 24	1 ( 0.9%)	0	1 ( 0.5%)
Responder, n(%)	30 ( 27.8%)	50 ( 46.3%)	80 ( 37.0%)
95% Exact CI	0.1959, 0.3722	0.3665, 0.5615	0.3058, 0.4385
Noninferior Proportion Difference - Unstratified CMH Method (95% CI)	-0.03 (-0.14, 0.07)		
Superior Proportion Difference - Unstratified CMH Method (95% CI)	-0.19 (-0.31, -0.06)		
Superior Proportion Difference - Unstratified Exact Method (95% CI)	-0.19 (-0.32, -0.05)		
Non-Responder, n(%)	78 ( 72.2%)	58 ( 53.7%)	136 ( 63.0%)
Last participation date < Day 162 in DB phase	16 ( 14.8%)	1 ( 0.9%)	17 ( 7.9%)
Last participation date >= Day 162 and TSS at Week 24 not available	2 ( 1.9%)	6 ( 5.6%)	8 ( 3.7%)
>0% increase from baseline at Week 24	26 ( 24.1%)	18 ( 16.7%)	44 ( 20.4%)
<50% reduction from baseline week 24	59 ( 54.6%)	51 ( 47.2%)	110 ( 50.9%)

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;

Source: Listing 16.3.2.1

Data Extracted: CRF data: 01JUL2019

Source: ...\\version5\\prog\\t-tss24.sas v9.4 Output file: t-tss24.out 09MAR2021:19:52

Table 15.12.2.1: Analysis of Response Rate in TSS at Week 24 and at Week 12  
Double-Blind Phase  
Intent-to-Treat Analysis Set

	MMB (N=215)	RUX (N=217)	Total (N=432)
<hr/>			
Response Rate of Total Symptom Score at Week 12			
Subjects Evaluable at Week 12, n	108	108	216
TSS = 0 at baseline and TSS >0 or missing at Week 12	1 ( 0.9%)	0	1 ( 0.5%)
Responder, n(%)	28 ( 25.9%)	43 ( 39.8%)	71 ( 32.9%)
95% Exact CI	0.1797, 0.3525	0.3052, 0.4968	0.2665, 0.3957
Noninferior Proportion Difference - Unstratified CMH Method (95% CI)	-0.01 (-0.11, 0.10)		
Superior Proportion Difference - Unstratified CMH Method (95% CI)	-0.14 (-0.26, -0.01)		
Superior Proportion Difference - Unstratified Exact Method (95% CI)	-0.14 (-0.27, -0.00)		
Non-Responder, n(%)	80 ( 74.1%)	65 ( 60.2%)	145 ( 67.1%)
Last participation date < Day 78 in DB phase	7 ( 6.5%)	1 ( 0.9%)	8 ( 3.7%)
Last participation date >= Day 78 and TSS at Week 12 not available	4 ( 3.7%)	4 ( 3.7%)	8 ( 3.7%)
>0% increase from baseline at Week 12	24 ( 22.2%)	17 ( 15.7%)	41 ( 19.0%)
<50% reduction from baseline at week 12	68 ( 63.0%)	60 ( 55.6%)	128 ( 59.3%)

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;

Source: Listing 16.3.2.1

Data Extracted: CRF data: 01JUL2019

Source: ...\\version5\\prog\\t-tss24.sas v9.4 Output file: t-tss24.out 09MAR2021:19:52

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