Relapse rate at 6, 12 and 18 months

Response assessment		Relapse		No Relapse		Comparison of treatment arms
Time	**Arm**	**N**	**% [LL;UL]**	**N**	**% [LL;UL]**	**p-Value**
≤ 24 weeks	Eltrombopag	5	16.7 [6.9; 34]	25	83.3 [66; 93.1]	0.024
	Placebo	0	n.a.	31	100 [86.9; 100]	
≤ 48 weeks	A	3	10.7 [2.9; 28]	25	89.3 [72; 97.1]	
	B	1	50 [9.5; 90.5]	1	50 [9.5; 90.5]	
	C	10	38.5 [22.4; 57.5]	16	61.5 [42.5; 77.6]	
	D	0	n.a.	4	100 [45.4; 100]	
	Other	0	n.a.	1	100 [16.7; 100]	
≤ 72 weeks	A1	1	50 [9.5; 90.5]	1	50 [9.5; 90.5]	
	A2	9	34.6 [19.3; 53.9]	17	65.4 [46.1; 80.7]	
	B	1	50 [9.5; 90.5]	1	50 [9.5; 90.5]	
	C	10	38.5 [22.4; 57.5]	16	61.5 [42.5; 77.6]	
	D	0	n.a.	4	100 [45.4; 100]	
	Other	0	n.a.	1	100 [16.7; 100]	

Clinical relapse is considered as the occurrence of any of the following events in a patient who had shown a hematological response (CR or PR or single lineage response):

- meeting again the criteria for MAA
- renewed transfusion requirement
- decrease in any of the responded peripheral blood counts to the pre-study baseline

To qualify as a relapse, peripheral blood count decrease has to be demonstrated for a minimum of two times over a period of 2 weeks.

Patients who withdrew consent or dropped out after hematological response before the respective visit will be counted as “missing” in descriptive tables. Proportions of relapse will be estimated at 6, 12 and 18 months per treatment arm / group. 95% confidence limits according to Agresti and Coull for the proportions of relapse will be computed.

Only after 6 months from therapy start, the null hypothesis that the OR is 1 will be assessed by Fisher’s exact test. The hypothesis will be tested against two-sided alternative at the 5% level of significance. A 95% confidence interval for the OR will be provided.

A cox regression will be performed to quantify the relationship between relapse and total dose of Eltrombopag administered. “Age” and “disease severity” will be included in the regression model.