Primary endpoint: Change in measure response between time point Baseline and time point 6 months

Primary analysis of the primary endpoint

The primary endpoint of the study is the (trilineage) hematologic response rate (CR + PR) at 6 months. The response assessment will be calculated as defined by the response criteria according tot he protocol. Additionally, the investigator will classify hematologic response. Both assessments will be evaluated descriptively, but the calculated assessment will be prevailing for hypothesis testing.

The analysis of the primary endpoint will be based on PPS. Absolute and relative frequency of patients with response (CR or PR) will be derived by treatment arm.

Let the counts in the true population denote

Treatment	Response yes	Response no	
Eltrombopag	n_{11}	n_{11}	
Placebo	n_{21}	n_{21}	

If the hematological response is independent of the treatment arm the odds ratio ($OR=n_{11}*n_{22}/(n_{12}*n_{21})$.) will be 1. Thus, Fisher's exact test may be interpreted as a test of the OR and is suitable for comparing the frequencies between treatment arms. The null hypothesis

H0: OR=1

will be tested against the one-sided alternative

H1: OR > 1

The null hypothesis will be rejected if the p-value is below 0.05. A lower 95% confidence limit for the OR will be provided. Additionally, 95% confidence limits for the proportions of responses in each treatment arm will be generated using the method proposed by Agresti and Coull.

Results:

	Eltrombopag N= 35		**Placebo N= 40**		**Comparison of treatment arms**		
Response	**N**	**% [95% CI]**	**N**	**% [95% CI]**	**p-value**	**OR**	**95% LL for OR**
CR	4	11.4 [3.9; 26.5]	2	5 [NA; NA]			
PR	21	60 [43.5; 74.5]	15	37.5 [24.2; 53]			
NR	10	28.6 [16.2; 45.2]	23	57.5 [42.2; 71.5]			
Yes (CR or PR)	25	71.4 [54.8; 83.8]	17	42.5 [28.5; 57.8]	0.011	3.325	1.354
No (NR)	10	28.6 [16.2; 45.2]	23	57.5 [42.2; 71.5]			