

Demographic and Baseline characteristics

Selected demographic and baseline clinical characteristics (FAS)

Patient characteristics			Eltrombopag N=41	Placebo N=44	Total N=85
Sex	Male	n (%)	23 (56.1%)	22 (50.0%)	45 (52.9%)
	Female	n (%)	18 (43.9%)	22 (50.0%)	40 (47.1%)
Ethnicity	Caucasian	n (%)	35 (85.4%)	39 (88.6%)	74 (87.1%)
	Asian	n (%)	2 (4.9%)	1 (2.3%)	3 (3.5%)
	Other	n (%)	4 (9.8%)	4 (9.1%)	8 (9.4%)
Age (years)	Arithmetic mean / STD		51.9 / 18.0	49.8 / 19.2	50.8 / 18.5
	Median (IQR)		55.0 (35.0-65.0)	46.5 (35.5-65.2)	53.0 (35.0-65.0)
	Range		20 - 84	19 - 83	19 - 84
	18-60 years	n (%)	25 (61.0%)	28 (63.6%)	53 (62.4%)
	> 60 years	n (%)	16 (39.0%)	16 (36.4%)	32 (37.6%)
Transfusion-dependency	yes	n (%)	27 (65.9%)	22 (50.0%)	49 (57.6%)
GPI deficient erythrocytes (%)	Arithmetic mean / STD		1.7 / 5.0	1.4 / 2.8	1.6 / 4.0
	Median (IQR)		0.0 (0.0-0.2)	0.1 (0.0-1.0)	0.1 (0.0-0.7)
	Range		0 - 23	0 - 10	0 - 23
GPI deficient granulocytes* (%)	Arithmetic mean / STD		7.7 / 16.8	9.0 / 16.2	8.4 / 16.3
	Median (IQR)		0.2 (0.0-1.6)	0.8 (0.1-8.5)	0.5 (0.0-8.0)
	Range		0 - 65	0 - 65	0 - 65
Hemoglobin (g/L)	Arithmetic mean / STD		90.4 / 12.9	89.1 / 14.1	89.8 / 13.5
	Median (IQR)		90.0 (85.0 - 98.0)	93.7 (80.2 - 100.0)	90.0 (82.1 - 98.0)
	Range		64 - 124	65 - 117	64 - 124
Reticulocytes (g/L)	Arithmetic mean / STD		50.1 / 27.5	56.0 / 21.8	53.2 / 24.7
	Median (IQR)		41.7 (28.2 - 76.2)	54.0 (40.8 - 71.2)	52.8 (34.6 - 76.2)
	Range		5 - 106	17 - 101	5 - 106
ANC (10 ⁹ /L)	Arithmetic mean / STD		1.04 / 0.44	1.14 / 0.70	1.09 / 0.59
	Median (IQR)		1.07 (0.64 - 1.29)	0.90 (0.64 - 1.42)	0.98 (0.64 - 1.29)
	Range		0.0 - 2.1	0.1 - 3.3	0.0 - 3.3
Platelet count (10 ⁹ /L)	Arithmetic mean / STD		24.5 / 20.2	30.0 / 32.7	27.4 / 27.4
	Median (IQR)		20.0 (12.0-27.0)	23.0 (18.5-33.2)	22.0 (14.0-29.0)
	Range		4 - 116	2 - 222	2 - 222
Number of platelet concentrate transfusions*	Arithmetic mean / STD		3.7 / 3.3	5.1 / 5.9	4.4 / 4.6
	Median (IQR)		2.0 (1.2-5.5)	3.0 (1.0-7.5)	2.0 (1.0-6.0)
	Range		1 - 12	0 - 22	0 - 22
Number of packed red blood cell transfusions*	Arithmetic mean / STD		4.4 / 4.1	4.2 / 2.2	4.3 / 3.3
	Median (IQR)		3.0 (2.0-4.8)	4.0 (2.0-6.0)	4.0 (2.0-6.0)
	Range		1 - 18	1 - 9	1 - 18
Number of transfusions since diagnosis*	Arithmetic mean / STD		6.4 / 6.4	7.4 / 7.0	6.9 / 6.6
	Median (IQR)		4.0 (2.0-7.0)	5.0 (2.0-10.0)	4.0 (2.0-8.0)
	Range		1 - 22	1 - 28	1 - 28

*subset of patients transfused

Baseline medical history and anamnesis (FAS)

Number of patients n (%)	Eltrombopag N=41	Placebo N=44	Total N=85
Medical history			
Cardiovascular events	12 (29.3%)	18 (40.9%)	30 (35.3%)
Neurological disorders / events	5 (12.2%)	10 (22.7%)	15 (17.6%)
Endocrine disorders	9 (22.0%)	12 (27.3%)	21 (24.7%)
Gastrointestinal and hepato-pancreato-biliary events	10 (24.4%)	7 (15.9%)	17 (20.0%)
Persistent or serious infections	2 (4.9%)	1 (2.3%)	3 (3.5%)
Hematologic / oncologic disease	13 (31.7%)	14 (31.8%)	27 (31.8%)
Musculoskeletal disorders	9 (22.0%)	12 (27.3%)	21 (24.7%)
Surgeries	16 (39.0%)	18 (40.9%)	34 (40.0%)
Renal and urinary tract events	4 (9.8%)	5 (11.4%)	9 (10.6%)
Autoimmune disease	11 (26.8%)	11 (25.0%)	22 (25.9%)
Pulmonary disease	5 (12.2%)	6 (13.6%)	11 (12.9%)
Thromboembolic events	3 (7.3%)	5 (11.4%)	8 (9.4%)
Familial history of hematologic / oncological disease	11 (26.8%)	8 (18.2%)	19 (22.4%)
Anamnesis			
Anemia signs in the last 12 weeks	22 (53.7%)	26 (59.1%)	48 (56.5%)
Bleeding signs in the last 12 weeks	11 (26.8%)	19 (43.2%)	30 (35.3%)
Hemolysis signs in the last 12 weeks	2 (4.9%)	1 (2.3%)	3 (3.5%)
Infections in the last 12 weeks	7 (17.1%)	6 (13.6%)	13 (15.3%)
Concomitant medication	29 (70.7%)	35 (79.5%)	64 (75.3%)
Transfusions in the last 12 weeks	27 (65.9%)	22 (50.0%)	49 (57.6%)