An open-label extension study of the long-term safety and efficacy of romiplostim in children with chronic immune thrombocytopenia

Patients

66 Children with chronic immune thrombocytopenia

34% 56%

- Median baseline age: 11 (3-18) years
- Platelet count: **28 (2-458)x10⁹/L**

Treatment

Subcutaneous **Romiplostim**

- Median average weekly dose: 4.8 (0.1-10) µg/kg

Median treatment duration: 2.6 (0.1-7.0) years

Results

	Patients
Treatment completation:	37/66 (56%)
Treatment discontinuation:	28/66 (42%)

Reasons

• Consent withdrawn	(n=10)	• Administrative decision	(n=2)
• Required other therapy	(n=5)	• Adverse events	(n=2)
• Non-compliance	(n=4)	• Other	(n=2)
• Per protocol	(n=3)		

Safety

The most common adverse events
- Headache
- Contusion

Efficacy

Median platelet counts
- **>50x10⁹/L** from week 2
- **>100x10⁹/L** from weeks 24 to 260

94% ≥1 platelet response
72% responded at 75% of visits
58% responded at 90% of visits

Treatment-free responses

15 Patients (23%) achieved a treatment-free response when romiplostim was withheld

6 9

• Platelet counts with no medications for ≥24 weeks	50x10⁹/L
• Median time with platelet counts >100x10⁹/L	46 (25-109) weeks
• Median immune thrombocytopenia duration	4 (1-12) years
• Median duration of romiplostim treatments	2 (1-6) years

Tarantino *et al.*, Haematologica, 2019