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

### Patients

<table>
<thead>
<tr>
<th>Children with chronic immune thrombocytopenia</th>
<th>66</th>
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</thead>
<tbody>
<tr>
<td>Median baseline age: 11 (3-18) years</td>
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<tr>
<td>Platelet count: 28 (2-458) x 10⁹/L</td>
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<tr>
<td>34%</td>
<td>56%</td>
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</tbody>
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### 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 completion: 37/66 (56%)
- Treatment discontinuation: 28/66 (42%)

#### Safety

- The most common adverse events:
  - Headache
  - Contusion

#### Efficacy

- Median platelet counts:
  - > 50 x 10⁹/L from week 2
  - > 100 x 10⁹/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
- Platelet counts with no medications for ≥ 24 weeks: 50 x 10⁹/L
- Median time with platelet counts > 100 x 10⁹/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*