

Neunert CE, Rose MJ. Romiplostim for the management of pediatric immune thrombocytopenia: drug development and current practice. *Blood Adv.* 2019 Jun 25;3(12):1907-1915. doi: 10.1182/bloodadvances.2019000279. PMID: 31239245

Background: Treatment for chronic immune thrombocytopenia (cITP) in children is largely limited to immunosuppressive agents. Thrombopoietin receptor agonists (TRAs) have been used to treat cITP in adults for over a decade.

Objective: To examine the safety and efficacy of the TRA, romiplostim in children with cITP.

Design: This pooled analysis combined data from pediatric patients across five clinical trials of romiplostim: two completed double-blind, placebo-controlled trials, two completed open-label extensions, and one ongoing open-label trial.

ACADEMIC P.E.A.R.L.S

Pediatric Evidence And Research Learning Snippet



Romiplostim: Thrompoietin Receptor Agonist - It's Efficacy in Treating Pediatric Chronic ITP

Results: Study population: 282 patients received romiplostim and 24 had placebo, majority had 1 prior treatment mostly corticosteroids. There was one bleeding episode in 58.7% of patients prior to Romiplostim therapy.

Treatment Duration & dosing: Among 282 patients receiving romiplostim, median treatment duration was 65 weeks (range 8-471 weeks) and median weekly dose was 6.6 µg/kg (range 0.1-9.7 µg/kg).

Response: Overall, 89% of Romiplostim treated patients had platelet responses. Nineteen patients (7%) maintained treatment-free responses for >6 months while withholding all ITP therapy.

Adverse events: Grade 3 and 4 adverse events of bleeding occurred in 10% and <1% of romiplostim-treated patients, respectively. Non bleeding adverse events seen were pyrexia, nasopharyngitis, cough, oropharyngeal pain, diarrhea, and upper abdominal pain.

Conclusion: Across five pediatric clinical trials, romiplostim was well tolerated. Most patients had a platelet response; some maintained responses for at least 6 months while withholding all ITP therapy.

Key Message: Romiplastim, once a week subcutaneous injection can be treatment option in children with chronic ITP.

EXPERT COMMENT



"Romiplostim was approved for pediatric use in Decmeber 2018 in patients >1 year of age with ITP > 6 months duration and insufficient response to corticosteroids. Not all patients with chronic ITP require treatment. Careful patient selection is important especially those having impaired quality of life due to low platelet counts. The data is still emerging and we are learning about the efficacy and adverse effects like bone marrow response, fibrosis and development of antibodies to Romiplostim. Romiplostim is being tried for various other off label indications like neonatal thrombocytopenia, hereditary thromobocytopenia etc. Indian data is needed to establish efficacy in our patients."

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Reference

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