



HC0033 Drugs for Pediatric ITP - List of included studies

Randomized Controlled Trials

1. Grainger JD, Blanchette VS, Grotzinger KM, Roy A, Bussel JB. Health-related quality of life in children with chronic immune thrombocytopenia treated with eltrombopag in the PETIT study. *Br J Haematol.* 2019;185(1):102-106.
2. Mathias SD, Li X, Eisen M, Carpenter N, Crosby RD, Blanchette VS. A Phase 3, Randomized, Double-Blind, Placebo-Controlled Study to Determine the Effect of Romiplostim on Health-Related Quality of Life in Children with Primary Immune Thrombocytopenia and Associated Burden in Their Parents. *Pediatr Blood Cancer.* 2016;63(7):1232-1237.
3. Tarantino MD, Bussel JB, Blanchette VS, et al. Romiplostim in children with immune thrombocytopenia: a phase 3, randomised, double-blind, placebo-controlled study. *Lancet.* 2016;388(10039):45-54.
4. Bussel JB, de Miguel PG, Despotovic JM, et al. Eltrombopag for the treatment of children with persistent and chronic immune thrombocytopenia (PETIT): a randomised, multicentre, placebo-controlled study. *Lancet Haematol.* 2015;2(8):e315-325.
5. Grainger JD, Locatelli F, Chotsampancharoen T, et al. Eltrombopag for children with chronic immune thrombocytopenia (PETIT2): a randomised, multicentre, placebo-controlled trial. *Lancet.* 2015;386(10004):1649-1658.
6. Klaassen RJ, Mathias SD, Buchanan G, et al. Pilot study of the effect of romiplostim on child health-related quality of life (HRQoL) and parental burden in immune thrombocytopenia (ITP). *Pediatr Blood Cancer.* 2012;58(3):395-398.
7. Bussel JB, Buchanan GR, Nugent DJ, et al. A randomized, double-blind study of romiplostim to determine its safety and efficacy in children with immune thrombocytopenia. *Blood.* 2011;118(1):28-36.
8. Elalfy MS, Abdelmaksoud AA, Eltonbary KY. Romiplostim in children with chronic refractory ITP: randomized placebo controlled study. *Ann Hematol.* 2011;90(11):1341-1344.

Prospective Comparative Cohort Studies

9. Tantawy AAG, Elsherif NHK, Ebeid FS, et al. Evaluation of the effect of eltrombopag therapy on the platelet collagen receptor glycoprotein VI (GPVI) expression and soluble GPVI levels in young patients with immune thrombocytopenia. *J Thromb Thrombolysis.* 2023;55(2):243-251.
10. Grace RF, Klaassen RJ, Shimano KA, et al. Fatigue in children and adolescents with immune thrombocytopenia. *Br J Haematol.* 2020;191(1):98-106.
11. Grace RF, Shimano KA, Bhat R, et al. Second-line treatments in children with immune thrombocytopenia: Effect on platelet count and patient-centered outcomes. *Am J Hematol.* 2019;94(7):741-750.