

Advancements in PEDIATRIC ITP: What You Need to Know



RAPID RECAP

Management Strategies for Pediatric ITP^{1,2}

Management of newly diagnosed ITP in children depends on:

- Severity of bleeding
- Risk factors
- Degree of thrombocytopenia (low platelet count)
- Quality of life
- Preferences of the patient, caregiver, and family

Initial treatment options:

- **Watchful waiting** (no immediate treatment)
- **Pharmacologic treatments**, including:
 - Intravenous immune globulin (IVIG)
 - Anti-D immunoglobulin
 - Glucocorticoids

ASH 2019 Guidelines for ITP¹

- In children with **newly diagnosed ITP and no or minor bleeding**, the panel suggests observation rather than corticosteroids (*conditional recommendation based on very low certainty in the evidence*)
- In children with **newly diagnosed ITP and no or minor bleeding**, the panel recommends observation rather than:
 - Intravenous immunoglobulin (*strong recommendation based on moderate certainty in the evidence*)
 - Rather than anti-d immunoglobulin (*strong recommendation based on moderate certainty in the evidence*)
- In children with **ITP who are unresponsive to first-line treatment**, the panel suggests the use of (*all conditional recommendations based on very low confidence in the evidence*):
 - TPO-RAs (thrombopoietin receptor agonist) rather than rituximab
 - TPO-RAs rather than splenectomy
 - Rituximab rather than splenectomy

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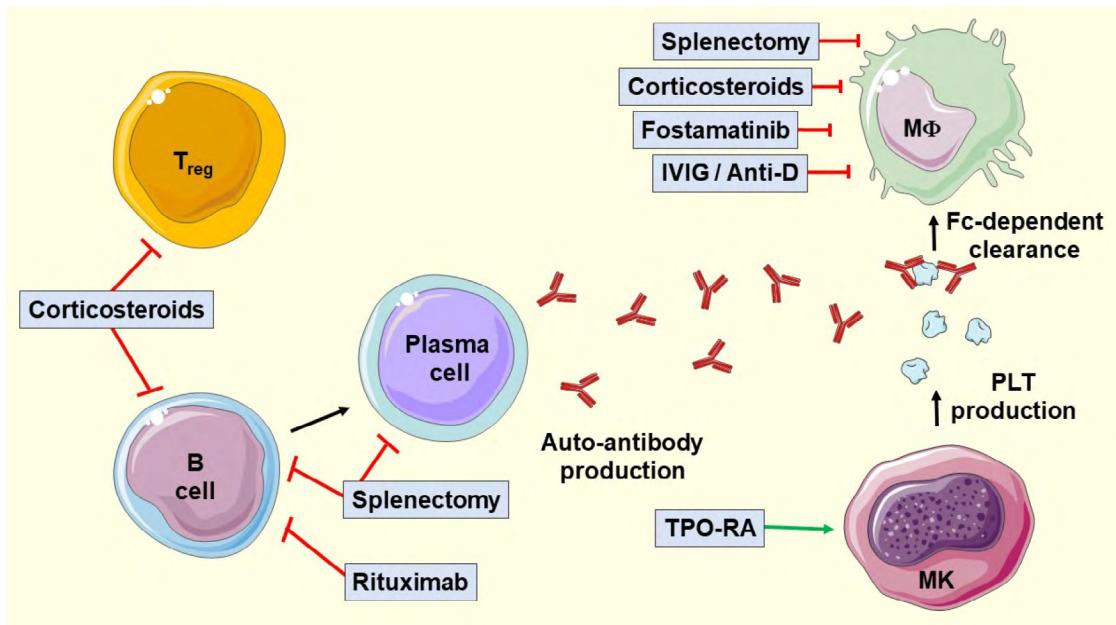


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Therapies Under Investigation for Pediatric ITP³

Name	Drug Class/MoA	Phase	Administration Route
Avatrombopag	TPO-RA	3	Oral (tablet)
Rilzabrutinib	BTK inhibitor	3	Oral
Hetrombopag*	TPO-RA	3	Oral
Daratumumab	Anti-CD38 antibody	2	Injection
Obinutuzumab	Anti-CD20 antibody	2	Injection

Mechanism of Action of Available and Novel Agents for Pediatric ITP⁴⁻⁷



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Key Takeaways

- Personalized treatment selection is critical in pediatric ITP, guided by bleeding risk and patient/family preferences
- TPO-RAs offer durable, steroid-sparing options and are increasingly used earlier in the treatment pathway
- Monitoring strategies must assess not just platelet counts but also bleeding, treatment side effects, and health-related quality of life
- Shared decision-making and ongoing risk-benefit assessment are essential, especially when considering long-term therapy or splenectomy

References

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4. Martínez-Carballera D, Bernardo Á, Caro A, et al. [Treatment of Immune Thrombocytopenia: Contextualization from a Historical Perspective](#). *Hematol Rep.* 2024;16(3):390-412.
5. Strüßmann T, Jung J, Heinz J, et al. [Long-term complete remission of refractory severe idiopathic immune thrombocytopenia \(ITP\) treated with daratumumab](#). *Ann Hematol.* 2023;102(1):245-247.
6. Kuter DJ, Bussel JB, Ghanima W, et al. [Rilzabrutinib versus placebo in adults and adolescents with persistent or chronic immune thrombocytopenia: LUNA 3 phase III study](#). *Ther Adv Hematol.* 2023;14:20406207231205431.
7. Langrish CL, Bradshaw JM, Francesco MR, et al. [Preclinical efficacy and anti-inflammatory mechanisms of action of the Bruton tyrosine kinase inhibitor rilzabrutinib for immune-mediated disease](#). *J Immunol.* 2021;206(7):1454-1468.