AJPMHS GLOBAL RESEARCH NETWORK

AMERICAN Journal of Pediatric Medicine and Health Sciences
Volume 3, Issue 12, 2025 ISSN (E): 2993-2149

Modern Methods of Treatment for Idiopathic (Immune) Thrombocytopenic Purpura (ITP)

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Abstract: Idiopathic (immune) thrombocytopenic purpura (ITP) is an autoimmune disorder characterized by isolated thrombocytopenia, leading to increased risk of bleeding. Despite decades of use of corticosteroids, intravenous immunoglobulin (IVIG), and splenectomy, many patients experience relapse or become dependent on long-term therapy. In recent years, novel treatments – particularly thrombopoietin-receptor agonists (TPO-RAs), monoclonal antibodies (e.g., anti-CD20), and small-molecule inhibitors – have expanded therapeutic options. This review synthesises data from randomized trials, cohort studies, meta-analyses, and clinical guidelines published between 2015 and 2024, evaluating both efficacy and safety of traditional and emerging therapies. Findings reveal that TPO-RAs achieve durable platelet response in a majority of chronic ITP patients; monoclonal antibody therapies and splenectomy remain important in selected cases. Emerging agents show promise but require further long-term evaluation. The review highlights the importance of individualized therapy and outlines future research directions.

Keywords: Immune thrombocytopenic purpura, ITP, thrombopoietin-receptor agonists, rituximab, splenectomy, IVIG, chronic ITP, treatment guidelines.

INTRODUCTION

Idiopathic (immune) thrombocytopenic purpura (ITP) is an autoimmune disorder characterized by isolated thrombocytopenia caused by both increased destruction of platelets and impaired platelet production. Historically, conventional treatments for ITP have focused on suppressing the immune response or removing the spleen (e.g., with glucocorticoids, intravenous immunoglobulins (IVIG), splenectomy, and anti-CD20 therapies such as Rituximab).

However, ITP is a heterogeneous disease with unpredictable clinical course, and up to 75% of adult patients may develop a chronic form, which significantly affects their quality of life. These limitations in long-term efficacy, variability of patient responses, and potential side effects of immunosuppressive therapies have spurred research into novel, more targeted treatment methods.

Recently, the therapeutic paradigm in ITP management has shifted toward agents that stimulate platelet production or modulate specific immune pathways, rather than solely dampening immune-mediated platelet destruction. Among these, thrombopoietin-receptor agonists (TPO-RAs) such as Romiplostim and Eltrombopag have shown significant capacity to raise platelet counts in chronic ITP patients.

Moreover, newer therapeutic avenues are under active investigation, including small-molecule inhibitors of spleen tyrosine kinase (e.g., Fostamatinib), neonatal Fc receptor (FcRn) blockers, B-cell and plasma-cell targeted therapies, complement inhibitors, and other immunomodulatory agents.

Given the evolving landscape of ITP therapy, there is an urgent need for a comprehensive evaluation of modern treatment modalities – their mechanisms of action, efficacy, safety, and place in current clinical practice. The present paper aims to review and critically analyze current and emerging methods for the treatment of ITP, and discuss how these innovations may improve long-term outcomes for patients.

RESULTS

A total of 62 studies met the inclusion criteria. These included 14 randomised controlled trials, 22 cohort studies, 8 case–control studies, 12 systematic reviews or meta-analyses, and 6 international clinical guidelines. The studies collectively represented treatment outcomes in both adult and paediatric populations, with sample sizes ranging from 30 to more than 2,000 participants.

Most studies reported that corticosteroids remained the primary first-line treatment for newly diagnosed ITP. Prednisone and dexamethasone achieved early platelet responses ($\geq 30\times10^9$/L) in 65–85% of patients within 7–14 days. However, sustained responses at 6 months were observed in only 20–30% of cases.

Intravenous immunoglobulin (IVIG) produced rapid increases in platelet counts within 24–48 hours in 70–90% of patients, particularly useful for severe thrombocytopenia or active bleeding. Nevertheless, the effect was temporary, and platelet levels frequently declined within 2–4 weeks.

Fostamatinib, a spleen tyrosine kinase (SYK) inhibitor, produced stable platelet responses in 18–43% of heavily pretreated patients.

New biological agents, including FcRn antagonists (e.g., efgartigimod) and novel TPO-RAs, demonstrated promising preliminary results in early-phase clinical trials.

Gene-modulating and tolerance-inducing therapies are under investigation, but long-term data remain limited. Corticosteroids were associated with significant adverse effects with prolonged use, including hypertension, glucose intolerance, mood changes, and weight gain.

TPO-RAs demonstrated favourable tolerability, although some studies reported hepatotoxicity (eltrombopag) and thrombotic complications.

Rituximab caused infusion-related reactions and increased susceptibility to infections. Splenectomy carried risks of bleeding, sepsis, and long-term vulnerability to encapsulated bacterial infections.

DISCUSSION

The results indicate that while traditional first-line therapies such as corticosteroids and IVIG remain essential for rapid platelet elevation, their long-term effectiveness is limited. This supports the shift in modern clinical practice toward incorporating second-line and targeted therapies earlier in the treatment algorithm.

TPO-RAs have emerged as a central component of chronic ITP management due to their high response rates, favourable safety profiles, and ability to maintain sustained platelet counts with

continuous use. The data suggest that these agents significantly reduce bleeding risk and improve quality of life for patients who previously had limited options.

The reviewed evidence aligns closely with guidelines from the American Society of Hematology (ASH) and the International Consensus Report, both of which advocate short courses of corticosteroids for initial treatment and recommend TPO-RAs or rituximab before considering splenectomy. The decreasing use of splenectomy reflects a broader trend toward less invasive, immunologically targeted approaches.

Clinicians increasingly individualise treatment choices based on patient age, comorbidities, bleeding risk, and preference. Rapid-acting therapies such as IVIG remain crucial in emergency scenarios, whereas chronic cases benefit from sustained, low-toxicity options such as TPO-RAs. Rituximab remains valuable for patients seeking finite therapy rather than long-term medication.

Emerging targeted therapies, including SYK inhibitors and FcRn antagonists, represent significant progress toward precision medicine in ITP management. These agents offer alternatives for patients with multi-refractory disease and may eventually redefine second-line treatment standards. Future research should prioritise long-term comparative studies, patient-reported outcomes, combination therapy regimens, and biomarkers predicting response to specific treatments. A growing interest in immune tolerance–inducing strategies may eventually lead to curative approaches.

The heterogeneity of study designs and outcome measures limited the ability to perform quantitative comparisons. Many studies lacked long-term follow-up, and paediatric representation was weaker than adult research. As a narrative review, findings depend on the quality and completeness of the included studies.

CONCLUSION

The landscape of ITP treatment has evolved significantly. While corticosteroids and IVIG remain essential for rapid control of acute bleeding, their long-term limitations – relapse risk, dependency, and toxicity – make them suboptimal for chronic management. Thrombopoietin-receptor agonists (e.g., eltrombopag, romiplostim) now represent a cornerstone of second-line and maintenance therapy, providing high rates of durable platelet response with acceptable safety profiles. Monoclonal antibody therapy (anti-CD20) continues to be a useful alternative for patients seeking finite, non-surgical therapy, and surgical splenectomy, though effective, is increasingly reserved for refractory cases. Emerging therapies – including small-molecule inhibitors and novel biologics – may further expand options, especially for multi-refractory ITP. Future research should prioritise long-term outcomes, quality-of-life assessment, direct comparisons of available agents, and personalized treatment algorithms tailored to patient risk, preferences, and comorbidities.

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