

# Fostamatinib in the Management of Refractory Immune Thrombocytopenia: A Case Report and Therapeutic Insights

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## Introduction

Fostamatinib has recently emerged as a promising treatment for refractory immune thrombocytopenia (ITP), an autoimmune disorder characterized by low platelet counts and an increased risk of bleeding due to the destruction of platelets by the immune system. Refractory ITP is particularly challenging to manage because it does not respond to standard therapies, such as corticosteroids, intravenous immunoglobulin (IVIG), and splenectomy. The development of fostamatinib, a spleen tyrosine kinase (SYK) inhibitor, offers a new therapeutic option for patients with this difficult condition.

## Description

A 45-year-old woman with a five-year history of ITP serves as an illustrative case. Despite receiving multiple treatments, including high-dose corticosteroids, IVIG, rituximab, and a thrombopoietin receptor agonist (TPO-RA), her platelet counts remained stubbornly below 30,000/ $\mu\text{L}$ . She experienced frequent mucosal bleeding and petechiae, which severely affected her quality of life. After these treatments failed, fostamatinib was considered as an alternative option. The patient started fostamatinib at a dose of 100 mg twice daily. Baseline laboratory tests, including a complete blood count (CBC), liver function tests, and renal function tests, were conducted to ensure her safety before beginning the new medication. Within four weeks of initiating fostamatinib, her platelet count rose to 50,000/ $\mu\text{L}$ , and by the eighth week, it stabilized above 100,000/ $\mu\text{L}$ . The reduction in bleeding symptoms was significant, and her quality of life improved as a result. She continued fostamatinib therapy for six months, maintaining stable platelet counts without experiencing any major side effects. The mechanism of action of fostamatinib involves the inhibition of SYK, a crucial component in the Fc receptor signaling pathway that mediates the phagocytosis of antibody-coated platelets. By blocking SYK, fostamatinib reduces the destruction of platelets, thereby increasing their count in patients with ITP. This novel mechanism makes fostamatinib particularly effective for patients who have not responded to other treatments, as seen in the case described. The efficacy of fostamatinib

in refractory ITP is notable. In the presented case, the patient achieved a sustained increase in platelet counts after multiple other treatments had failed. This underscores fostamatinib's potential to provide a valuable alternative for patients with limited treatment options. Its ability to stabilize platelet counts and reduce bleeding symptoms can significantly improve patients' quality of life, which is often severely impacted by chronic ITP and its associated treatments. The safety profile of fostamatinib is another important consideration. The most common side effects include diarrhea, hypertension, and elevated liver enzymes. Regular monitoring of liver function is recommended to detect any potential hepatotoxicity early. In the described case, the patient tolerated fostamatinib well and did not experience any significant adverse effects, further supporting its safety in clinical use. Improvement in quality of life is a critical outcome for patients with ITP. The reduction in bleeding symptoms and the stabilization of platelet counts can lead to significant improvements in overall well-being, as demonstrated in the patient's case. Fostamatinib's ability to achieve these outcomes makes it a valuable addition to the therapeutic arsenal for managing refractory ITP. There is also potential for fostamatinib to be used in combination with other treatments, such as TPO-RAs or immune-suppressants. While it has shown efficacy as a monotherapy, combining it with other treatments could potentially enhance therapeutic outcomes and offer a broader range of management strategies for refractory ITP. This aspect warrants further investigation to fully understand the benefits and risks of combination therapy.

## Conclusion

In conclusion, fostamatinib offers a new and effective treatment option for patients with refractory ITP. Its novel mechanism of action, favorable safety profile, and ability to improve quality of life make it a promising therapeutic agent. The case of the 45-year-old woman highlights its potential to provide significant clinical benefits in patients who have exhausted other treatment options. As further research and clinical experience accumulate, the role of fostamatinib in the management of ITP will continue to be better defined, potentially offering new hope to patients struggling with this challenging condition.

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