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Commentary

Fostamatinib in the Treatment of Primary Immune Thrombocytopenia

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DESCRIPTION

Fostamatinib, a recently approved SYK (Spleen Tyrosine Kinase) inhibitor, has demonstrated its safety and efficacy in treating adult primary Immune Thrombocytopenia (ITP). However, translating clinical trial outcomes into real-world clinical practice often presents challenges. This study evaluates the real-world effectiveness and safety of fostamatinib in ITP patients across 42 Spanish centers, including both primary and secondary cases. The study cohort consisted of patients with a median age of 66 years (Interquartile Range [IQR], 56-80 years), and 55.8% of the participants were women. The median duration from ITP diagnosis to fostamatinib initiation was 51 months (IQR, 10-166 months), indicating a considerable duration of disease before the introduction of fostamatinib. Prior to fostamatinib treatment, patients had received a median of four therapies (IQR, 2-5). These therapies included eltrombopag (76.1%), romiplostim (57.2%), and Intravenous Immunoglobulins (IVIG) (44.2%). Such extensive prior treatment underscores the chronic nature and treatment-refractory characteristics of the ITP patient population involved in this study. Notably, 42% of patients exhibited signs or symptoms of bleeding in the month preceding the initiation of fostamatinib therapy, highlighting the urgent need for effective treatment options.

Efficacy of fostamatinib

The results from this study indicate that fostamatinib is highly effective in managing ITP. A substantial 79.0% of patients responded positively to fostamatinib treatment. Among these responders, 53.6% achieved a complete response, defined as a platelet count greater than 100 × 10^9/L. These response rates are particularly encouraging, given the heavily pre-treated nature of the cohort.

Monotherapy efficacy: 60% of patients received fostamatinib as a monotherapy, achieving an impressive response rate of 85.4%. This suggests that fostamatinib alone can be a potent treatment option for many patients with ITP, potentially reducing the need for combination therapies and their associated complexities.

Time to response: The median time to achieve a platelet response was notably rapid at 11 days (IQR, 7-21 days). This quick response time is crucial in a clinical setting, particularly for patients presenting with bleeding symptoms who require prompt therapeutic intervention.

Duration of response: The proportion of time patients maintained a response over the 27-month study period was 83.3%. This prolonged duration of response indicates that fostamatinib not only provides rapid improvement in platelet counts but also sustains this response over a significant period, enhancing the long-term management of ITP.

Safety profile

Adverse events: The safety profile of fostamatinib observed in this real-world study aligns with previous clinical trials, with the majority of adverse events being mild to moderate (grade 1-2). Totally 48.5% of patients experienced adverse events, with the most common being diarrhea (n=28) and hypertension (n=21). These findings are consistent with the known side effects of fostamatinib, reflecting its manageable safety profile.

Serious adverse events: Serious adverse events were rare but noteworthy. One patient experienced deep venous thrombosis, and another developed an acute myocardial infarction. These events underscore the need for careful monitoring of patients on fostamatinib, particularly those with pre-existing risk factors for thrombotic events. The high response rates and rapid onset of action observed in this real-world study are comparable to those reported in clinical trials. However, real-world studies such as this one provide valuable insights that extend beyond the controlled environments of clinical trials. They offer a broader understanding of how a drug performs across diverse patient populations and varying clinical settings. The results of this study reinforce the role of fostamatinib as a potent and reliable treatment option for patients with ITP, including those who have failed multiple previous therapies. The high efficacy of fostamatinib as a monotherapy simplifies treatment regimens, potentially improving patient adherence and reducing the

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burden of managing combination therapies. Despite its strengths, this study has limitations that must be acknowledged. The retrospective nature of part of the data collection may introduce biases related to incomplete or inaccurate records. Additionally, the study's observational design means thatcausality cannot be firmly established, and the findings may not be generalizable to all ITP patients, particularly those with different demographic or clinical characteristics.

CONCLUSION

This real-world study of ITP patients treated with fostamatinib across 42 Spanish centers demonstrates that fostamatinib is both effective and safe for a wide range of patients, including those with primary and secondary ITP. The high response rates, rapid time to response, and sustained duration of response observed in this study affirm fostamatinib's role as a valuable treatment option in the management of ITP. These findings, coupled with a manageable safety profile, suggest that fostamatinib should be considered a frontline therapy for patients with ITP, particularly those who have exhausted other treatment options. Future research should focus on long-term outcomes and direct comparisons with other therapies to further establish fostamatinib's place in the therapeutic landscape for ITP. Additionally, studies involving larger and more diverse populations would help to generalize these findings and optimize treatment strategies for different patient subgroups. Nonetheless, this study provides strong evidence supporting the use of fostamatinib in real-world clinical practice, offering hope for improved management and quality of life for patients with ITP.