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POSTER ABSTRACTS

311.DISORDERS OF PLATELET NUMBER OR FUNCTION: CLINICAL AND EPIDEMIOLOGICAL

Efficacy and Safety of Fostamatinib for Immune Thrombocytopenia in Clinical Practice in Spain: Interim Results of Fostames, Our National Fostamatinib Registry

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Background: Fostamatinib is a splenic tyrosine kinase (SYK) inhibitor that prevents antibody-mediated platelet destruction. This drug has demonstrated to be effective and safe in immune thrombocytopenia (ITP). However, clinical trials may not accurately reflect what happens in clinical practice. Here we evaluated the efficacy and safety of eltrombopag in ITP in a real-world setting.
Aims: To assess the management of adult patients with immune thrombocytopenia (ITP) with fostamatinib (Tavlesse®) in routine clinical practice outside controlled clinical trials.
Methods: Multicentre, retrospective and prospective observational study at national level to evaluate the management of fostamatinib (Tavlesse®) in adult patients with ITP. An interim analysis of the medical records of all patients treated with fostamatinib (Tavlesse®) and suffering from ITP at each center was performed. Data were obtained in a retrospective and prospective observational, non-interventional manner to fulfil the exclusively scientific objective of this study, by conducting a review of the patient's clinical history. Thus, a total of 46 adult patients with ITP from 19 Spanish centers, who had been treated with fostamatinib, were evaluated.
Results: The median age of our cohort was 74 yr (interquartile range, IQR, 60-81 yr). 25/46 (54%) were women. 43 of them were Primary ITP, 2 Secondary ITP and only one Evans Syndrome. 20 (47.6%) had ≥ 1 comorbidity of the Charlson Comorbidity

Index. At the time of ITP diagnosis, the median platelet count was 8 x 10^9/L (IQR, 4-21 x 10^9/L) while 31 (69%) patients had hemorrhages.

The median time with a diagnosis of ITP at initiation of fostamatinib was 61 months (IQR, 9-160 months). The median number of therapies prior to fostamatinib was 4 (IQR, 3-6). Of the entire cohort of 46 patients, 35 had been exposed to eltrombopag, 33 to romiplostim, 23 to intravenous immunoglobulins (IVIG), and 21 to rituximab. Only eight and two patients had received avatrombopag and splenectomy prior to fostamatinib treatment, respectively.

26/46 patients (56.5%) were treated with fostamatinib monotherapy throughout ITP. Twenty-three patients (50%) had signs/symptoms of bleeding in the month prior to treatment initiation. The median platelet count at baseline on fostamatinib was 12 × 10^9/L (IQR, 6-28 × 10^9/L), while the median platelet count to best response achieved after fostamatinib treatment was 84 × 10^9/L (IQR, 28-187 × 10^9/L). The median time from initiation of fostamatinib to maximum response to treatment was 2 months (IQR, 0-3.25). Similarly, the median duration of treatment with fostamatinib was 2 months (IQR, 1-4.25). The median time from initiation of fostamatinib to dose increase to 150 mg twice a day was 16 days (IQR, 8-38).

A total of 32/46 patients (69.5%) responded to fostamatinib. Thus, 21 patients (45.7%) achieved a complete response (platelet count > 100 x 10^9/L) and 11 achieved a response. Twenty-one patients (45.6%) experienced adverse events, mainly grade 1-2, with diarrhea (n=13) and hypertension (n=8) being the most frequent. Twenty-five patients (54.3%) discontinued fostamatinib treatment: 18 did so due to inefficacy with three exitus due to severe bleeding in the context of refractory ITP. On the contrary, only three patients discontinued the drug due to toxicity/severe adverse events.

Summary/Conclusion: Fostamatinib was used in heavily treated patients. However, fostamatinib proved to be effective and well tolerated in unselected patients with primary and secondary ITP. The association of fostamatinib with other drugs or its use in earlier lines of therapy may be associated with higher platelet response rates.

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