Abstract: PB3323

Title: EFFICACY AND SAFETY OF FOSTAMATINIB FOR IMMUNE THROMBOCYTOPENIA IN CLINICAL PRACTICE IN SPAIN: RESULTS OF FOSTAMES, OUR NATIONAL FOSTAMATINIB REGISTRY

Abstract Type: Publication Only

Topic: Platelet disorders

Background:

Fostamatinib is a splenic tyrosine kinase (SYK) inhibitor that prevents antibody-mediated platelet destruction. This drug has been shown to be effective and safe in immune thrombocytopenia (ITP). However, clinical trials may not accurately reflect what happens in clinical practice. Here we evaluate the efficacy and safety of fostamatinib in ITP in a real-world scenario.

Aims:

To obtain real-world data on the treatment of adult patients with ITP with fostamatinib (Tavlesse®) in daily clinical practice outside of a clinical trial setting.

Methods:

Multicentre, retrospective and prospective observational study at national level to evaluate the use of fostamatinib (Tavlesse®) in adult patients with ITP. An 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 fulfill the exclusively scientific objective of this study, by conducting a review of the patient's clinical history.

Results:

A total of 138 adult patients with ITP from 42 Spanish centers, who had been treated with fostamatinib, were evaluated. 121 of them were primary ITP. The median age of our cohort (55.8% women) was 66 years (interquartile range, IQR, 56–80 years). At diagnosis, twenty-eight patients (20.2%) had a Charlson Comorbidity Index ≥ 1, the median platelet count was 13 x 109/L (IQR, 5-26 x 109/L) and ninety-five patients (68.8%) had hemorrhages.

When fostamatinib treatment was started, the median time since diagnosis of ITP was 51 months (IQR, 10–166 months). Seventeen were newly diagnosed ITP, twenty persistent ITP and one hundred and one chronic ITP. The median number of therapies prior to administration of fostamatinib was 4 (IQR, 2–5), including eltrombopag (76.1%), romiplostim (57.2%), intravenous immunoglobulins (IVIG) (44.2%), rituximab (29.0%) and avatrombopag (9.4%). Nineteen patients (13.8%) were splenectomized.

The median platelet count at the time of initiation of fostamatinib treatment was 21 x 109/L (IQR,8-47 x 109/L) while fifty-eight patients (42.0%) had signs/symptoms of bleeding in the month prior to treatment initiation. In our cohort, 85 patients (61.6%) increased fostamatinib to the maximum daily dose of 300 mg. The median time from initiation of fostamatinib to dose increase to 150 mg twice a day was 28 days (IQR, 16-39 days).

Seventy-nine percent 79.0% of patients responded to fostamatinib with 53.6% achieving a complete response (platelet count > 100 x 109/L). Eighty-three patients (60.1%) were treated with fostamatinib monotherapy throughout ITP achieved a response rate of 85.4%. The proportion of cumulative time spent in platelet response during the 27-month period examined was 83.3%. The median time to platelet response was 11 days (IQR, 7-21 days). Fostamatinib demonstrated higher responses in primary ITP (80.3%) than in secondary thrombocytopenia (68.7%). The platelet increase was sustained during the period under examination (Figure 1).

Sixty-seven patients (48.5%) experienced adverse events (AEs), mainly grade 1-2 with equal frequency in older (≥ 65 years of age) and younger patients; the most frequent AE was diarrhea (n = 28) and hypertension (n = 21). Ten patients developed neutropenia grade 3-4. One patient had deep venous thrombosis and one patient developed acute myocardial infarction.

Summary/Conclusion:

Fostamatinib was used in heavily treated ITP patients. However, even in such an inauspicious setting, fostamatinib was highly effective and presented a good safety profile in unselected patients with ITP.

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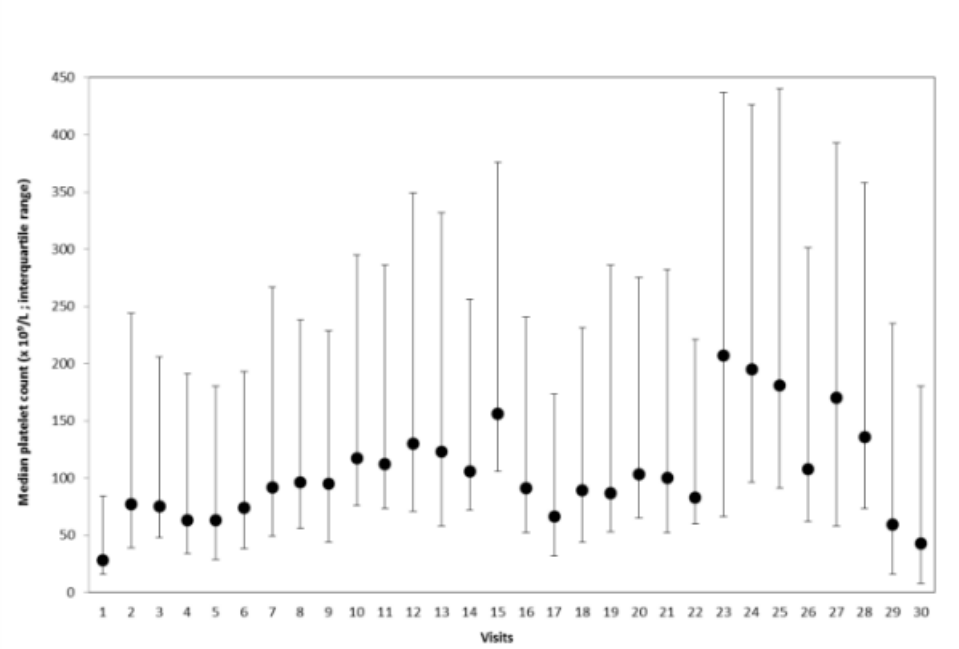

Figure 1: Platelet count over time.

Keywords: SYK, Autoimmune disease, Immune thrombocytopenia (ITP)