

RESPONSE AND CARDIOVASCULAR SAFETY OF FOSTAMATINIB IN CHRONIC IMMUNE THROMBOCYTOPENIA: A REAL WORLD ANALYSIS

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Background and Aims. Real-world evidence on fostamatinib (FST) in immune thrombocytopenia (ITP) has expanded substantially in recent years, with multicentre national experiences from Spain and Italy confirming its effectiveness and safety in routine clinical practice. Additional cohort analyses remain clinically relevant to better define treatment patterns and outcomes in patients with complex biological and clinical features, including autoimmune diathesis and cardiovascular/thrombotic risk. We therefore aimed to describe the characteristics of a real-world cohort of chronic ITP treated with FST, and to assess response rate, time to response, treatment exposure, and cardiovascular safety, while also exploring whether earlier response was associated with longer treatment exposure.

Methods. We retrospectively evaluated 29 patients with chronic ITP treated with FST in a real-world setting, with data cut-off on April 12, 2026. Outcomes of interest included overall response rate, time to response, treatment exposure, and cardiovascular safety. We also explored whether earlier response was associated with longer treatment exposure. Baseline patient and treatment characteristics are reported in Table 1.

Results. Baseline characteristics are summarized in Table 1. Briefly, the cohort showed a median TH2 score of 1, with only 4/29 patients (13.8%) displaying a hemorrhagic-risk profile, while the remainder had a prothrombotic profile; moreover, 18/29 patients (62.1%) had at least one cardiovascular risk factor. Overall response rate was 75.9% (22/29), including 15 complete responses and 7 partial responses, while 7 patients were non-responders. Among responders, median time to response was 15.5 days (IQR 10–42.5; range 3–409), with a median platelet count at response of 160×10^3/mmc. In heavily pretreated patients, overall response rate was 62.5% (5/8). At cut-off, 16 patients were still on treatment, with a median platelet count of 170×10^3/mmc (range 65–328); response was maintained in all but 2 patients who shifted from complete to partial response. No significant association was observed between shorter time to response and longer treatment exposure (Spearman rho=0.37, p=0.091). No new cardiovascular events were observed.

Conclusions. In this real-world chronic ITP cohort, fostamatinib showed high response rates and rapid activity, including in heavily pretreated patients. Outcomes were consistent with published real-world evidence. While early response did not predict longer treatment exposure, fostamatinib delivered meaningful and sustained benefit across this real-world cohort. Particularly reassuring was the absence of new cardiovascular events in a population enriched for cardiovascular risk factors.

Table 1. Baseline population characteristics

Characteristic	Value
Patients, n	29
Female sex, n (%)	21 (72.4)
Male sex, n (%)	8 (27.6)
Median age at diagnosis, years (range)	49 (11–84)
Median prior treatment lines, n	3
Heavily pretreated patients*, n (%)	8 (27.6)
Splenectomized patients, n (%)	7 (24.1)
Autoimmune profile	
Autoimmune diathesis, n (%)	14 (48.3)
Antiphospholipid antibodies positivity, n (%)	5 (17.2)
Cardiovascular/thrombotic profile	
Prior cardiovascular/thrombotic history, n (%)	5 (17.2)
– Ischemic heart disease, n (%)	2 (6.9)
– Deep vein thrombosis, n (%)	2 (6.9)
– Transient ischemic attack, n (%)	1 (3.4)
≥1 cardiovascular risk factor/comorbidity, n (%)	18 (62.1)
– Arterial hypertension, n (%)	11 (37.9)
– Hypercholesterolemia, n (%)	8 (27.6)
– Diabetes mellitus, n (%)	6 (20.7)
– Obesity, n (%)	5 (17.2)
– Smoking, n (%)	3 (10.3)
– Metabolic syndrome, n (%)	1 (3.4)
– Chronic heart failure, n (%)	1 (3.4)
– Chronic venous insufficiency, n (%)	1 (3.4)
– Abdominal aortic ectasia, n (%)	1 (3.4)
– Atrial fibrillation, n (%)	1 (3.4)
– Dilated cardiomyopathy, n (%)	1 (3.4)
TH2 score analysis	
Median TH2 score	1
Hemorrhagic-risk TH2 profile, n (%)	4 (13.8)
Prothrombotic TH2 profile, n (%)	25 (86.2)
Treatment characteristics	
Fostamatinib started for non-response, n (%)	21 (72.4)
Fostamatinib started for platelet count oscillation, n (%)	5 (17.2)
Fostamatinib started for adverse events to prior therapies, n (%)	3 (10.3)
Bridging treatment, n (%)	16 (55.2)

*Heavily pretreated defined as ≥4 prior treatment lines.