

Abstract: P2260

Title: REGIONAL DIFFERENCES IN HEMOPHILIA A TREATMENT APPROACHES: A COMPARISON OF PROPHYLAXIS TREATMENT USAGE BETWEEN THE US AND EUROPE

Abstract Type: e-Poster Presentation

Topic: Bleeding disorders (congenital and acquired)

Background:

The treatment of Hemophilia A patients is determined by a variety of factors. The choice of treatment - prophylaxis or on-demand - is influenced by the individual patient's response, disease severity, inhibitor presence, lifestyle, and treatment history.

Aims:

This study aims to investigate and compare the treatment approaches of Hemophilia A patients in the US versus EU4+UK (UK, France, Spain, Germany, and Italy). Additionally, this study also seeks to explore the reasons given by physicians for not utilizing prophylactic treatments using real-world data among patients diagnosed.

Methods:

The Ipsos Hemophilia Therapy Monitor, a multi-country quantitative online survey, was conducted from November 2022 to February 2023. A total of 246 physicians specializing in treating patients with hemophilia A from the US (n=70) and EU4+UK (n=176, including UK (n=24), France (n=30), Spain (n=40), Germany (n=41), and Italy (n=41) participated in the study. The study collected 1537 patient records from the participating physicians (US: 417, EU4+UK: 1120). Recruited from an access panel, treating physicians were screened for duration of practice in their specialty and hemophilia patient caseload.

Results:

The sampled US physicians (n=69) reported a significantly higher average percentage of Hemophilia A patients with inhibitors receiving on-demand treatment approach than the sampled EU4+UK physicians (n=158) (US: 37.1% vs EU4+UK: 28.2%, $p<0.05$). Equally, the sampled EU4+UK physicians reported a significantly higher average percentage of inhibitor patients receiving prophylaxis treatment (EU4+UK: 64.9% vs US: 54.1%, $p<0.05$).

The treatment approach for moderate and severe patients without inhibitors was more aligned across both regions, showing no significant statistical differences. The sampled EU4+UK physicians (n=173) reported an average percentage of 55.8% of their moderate hemophilia A patients without inhibitors receiving prophylaxis treatment, compared to 59.2% in the US (n=69). The average percentage for on-demand therapy was 39.7% in EU4+UK and 36.1% in the US.

For the severe patients without inhibitors, an average percentage of 72.0% in EU4+UK (n=175) and 67.4% in the US (n=69) receive prophylaxis treatment, whereas the average percentage for on-demand treatment was 24.1% in EU4+UK and 30.0% in the US.

In both sampled regions, the primary determinant for the lack of prophylactic treatment was attributable to patient or caregiver preference. However, secondary determinants exhibited significant regional variation. In the US, cost was a notable deterrent, impacting 22.2% of reported patients, followed by the consideration of patient's age, affecting 11.9% of the patients. In contrast, within the EU4+UK, disease severity emerged as the second most common determinant, with cost affecting a lower proportion of patients at 8.3%, and young age not factoring in as a determinant. The differences in cost and age considerations between the two regions are statistically significant ($p<0.01$).

Summary/Conclusion:

The study uncovered geographical variations in the treatment approach for hemophilia patients between the US and EU. In the US cohort, more Hemophilia A patients with inhibitors are on on-demand treatment compared to their EU counterparts. In the EU, a higher percentage of patients with inhibitors receive prophylaxis treatment. Patients' or caregivers' preferences were the primary reasons for not receiving prophylaxis in the US and EU, with expense and youth being significant factors in the US. Disease severity was the secondary common reason in the EU, suggesting distinct challenges in the healthcare markets of these regions.

Keywords: Prophylaxis, Treatment, Real world data, Hemophilia A