Abstract: S300

Title: ASSESSMENT OF JOINT HEALTH IN PATIENTS RECEIVING PROPHYLAXIS FOR HAEMOPHILIA A IN FIVE EUROPEAN COUNTRIES: A CROSS-SECTIONAL SURVEY

Abstract Type: Oral Presentation

Session Title: Advances in the science and care of persons with bleeding disorders

Background:

Goals for the optimal care of patients with haemophilia A (PwHA) include the prevention of musculoskeletal bleeding, which can lead to progressive joint damage and associated pain. However, real-world assessments of joint health in PwHA in the setting of current treatment options are limited.

Aims:

To evaluate joint health in patients with moderate/severe haemophilia A as reported by physicians and patients in five European countries.

Methods:

Retrospective analysis was performed on data from the Adelphi Real World Haemophilia Disease Specific Programme™, a multinational cross-sectional survey of haematologists and their patients. This analysis included physicians from France, Germany, Italy, Spain and the UK, and adult male patients with moderate/severe haemophilia A (baseline factor level ≤5%) currently receiving prophylaxis and without inhibitors. Data were collected from surveys completed between February 2020 and May 2021. Patients were considered to have haemophilia-affected joints (HAJs) if the physician reported one or more of the following: joint problems as a consequence of haemophilia; haemophilic arthropathy; synovitis/chronic inflammation in any joints; joint surgery due to haemophilia-related joint damage; ≥1 target joint (>3 bleeds in a joint during any 6 month period); most recent Pettersson score ≥1; or most recent Hemophilia Joint Health Score (HJHS) >3. Descriptive analyses and statistical tests (Fisher's exact; Student's t-test; Mann-Whitney U) were performed.

Results:

The study included 120 physicians and 351 patients with moderate (n=155)/severe (n=196) haemophilia A; 209 (59.5%) patients had HAJs and 142 (40.5%) had no HAJs. Of the 209 patients with HAJs, 135 (64.6%) had severe haemophilia. Patients with HAJs were older (mean [SD] age 34.7 [13.8] vs 29.0 [11.7] years) and had a higher frequency of prior inhibitors (19.6% vs 9.2%) compared to those without HAJs. Mean [SD] age at first presentation of joint damage was 23.9 [13.5] years and the HAJs were mostly of mild (52.2%) or moderate (36.6%) severity at last assessment. Patients received extended (23.0% and 16.2%) or standard half-life (44.5% and 58.5%) factor VIII treatment or non-factor therapy (32.5% and 25.4%) in the HAJ and non-HAJ groups, respectively. Pain/discomfort (any, i.e. not restricted to the joints) was significantly different between the two groups (p=0.0123) and was reported more frequently by patients in the HAJ group (85.7%) than the non-HAJ group (53.3%). A significantly greater proportion of patients with HAJs were receiving pain medication compared to those without HAJs (73.2% vs 60.6%; p=0.0144), including paracetamol (42.6% vs 23.9%; p=0.0004) and NSAIDs (37.8% vs 23.2%; p=0.0050). Up to half the patients with HAJs had a history of synovitis (49.8%), arthropathy (48.4%) or joint surgery (35.4%). In the HAJ group, the most recent mean [SD] overall Pettersson score and HJHS were 20.1 [19.4] and 42.6 [35.8], respectively. Patients with HAJs had a lower overall health status (mean [SD] EuroQol visual analogue scale score: 65.5 [19.3] vs 81.1 [14.6]; p=0.0089) compared to those without HAJs.

Summary/Conclusion:

In this study, 60% of patients with moderate/severe haemophilia A had HAJs. Individuals with HAJs were older, had higher rates of pain and pain medication use, and lower health-related quality of life compared to those without HAJs. These data, which reflect current clinical practice in Europe, indicate that patients with

moderate/severe haemophilia A experience HAJs despite prophylaxis and HAJs represent a clinically relevant burden for PwHA.

Keywords: Hemophilia A, Real world data, Quality of life, Prophylaxis