Reasons for Switching Factor Replacement Therapy Among Adults With Severe Haemophilia A and Haemophilia B in Europe

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INTRODUCTION

- Haemophilia A (HA) and haemophilia B (HB) are rare, recessive X-linked hereditary bleeding disorders caused by a deficiency of coagulation factor VIII (FVIII) and factor IX (FIX), respectively.
- Individuals with severe haemophilia (FVIII < 1% in HA or FIX < 1% in HB) can suffer recurrent, spontaneous bleeding into the joints and muscles, and therefore, require either on-demand treatment of bleeds or prophylaxis to prevent bleeding and preserve joint health (Emsteva 2015).
- Patients with haemophilia vary considerably with respect to bleeding phenotype, pharmacokinetic handling of factor, clinical response, and tolerability, emphasizing the importance of having available treatment options (Canavo 2015).
- Recent advancements in recombinant factor therapies offer patients the opportunity to switch to products considered safer or that provide better tolerability (Coppola 2016).
- Multiple recombinant FVIII (rFVIII) options are available in Europe but until recently, only one recombinant FIX product (rFIX) is available for HB in Europe.
- While patients with haemophilia appear to switch between treatment options, the reasons for switching are not well-described.

OBJECTIVES

- This study aimed to assess the reasons for switching treatments among individuals with severe HA or HB who are treated with factor replacement therapies in the real world, and to describe potential differences between the two groups with respect to the primary reasons for switching.
- In order to gain further insight into optimizing treatment for individuals with severe HB, the study also examined patient perceptions regarding methods for improving the provision of healthcare in this population.

METHODS

- This study assessed data from the Cost of Haemophilia across Europe – A Socioeconomic Survey (CHESS) study, the first comprehensive cost-of-illness study in adults with severe HA or HB in France, Germany, Italy, Spain, and the United Kingdom.
- In the CHESS study, 139 specialists completed an online survey between January – April 2015 and provided information on 12-month ambulatory and secondary care activity for 1,285 individuals; 551 patients completed corresponding patient questionnaires.
- In the present analysis, HA and HB participants were grouped by recombinant or plasma factor treatment.
- For HA, we assessed reasons for switching from a rFVIII to a pdFVIII or another rFVIII concentrate.
- For HB, we examined reasons for switching from a rFIX to a pdFIX or another rFIX concentrate.
- HB patient perceptions regarding how their care might be improved were also evaluated based on the patients’ responses to a survey checklist.

RESULTS

TREATMENT SWITCHES IN HEMOPHILIA A

- In the CHESS study, 46% of the total patient sample switched factor therapies due to lack of efficacy, tolerability, or development of an inhibitor.
- Primary Reasons for Switching Factor Concentrates Among 1,285 Patients With Severe Haemophilia A

TREATMENT SWITCHES IN HEMOPHILIA B

- The CHESS study collected data on 84 patients with severe HB who were treated with a rFIX, among whom treatment switches were reported in 14 (16.7%) patients.
- 13 of 14 patients (92.9%) had switched to a pdFIX; 1 patient switched to another rFIX.

CURRENT THERAPIES AND REASONS FOR SWITCHING AMONG PATIENTS PREVIOUSLY TREATED WITH RECOMBINANT HA OR HB THERAPIES

- A markedly greater proportion of individuals with severe HB (vs HA) switched to plasma options despite comparable clinical need for switching.

DISCUSSION

- In the CHESS study, individuals with severe HA and severe HB switched factor treatment for a variety of clinical reasons beyond access issues, including inhibitor development, poor tolerability, and lack of efficacy.
- The majority of individuals with severe HA or HB switched products to either other rFVIII therapies. By contrast, a minority of severe HB patients switched to another rFIX, likely due to the lack of access to alternative rFIX.
- Almost half of severe HB patients currently on rFIX expressed that the availability of new products would serve to improve their healthcare.
- Expanding access to new treatment options for patients may serve to enable an improvement in outcomes for individuals with severe HB.

DISECUSSIONS

- Results suggest that individuals with HA and HB switch treatments for efficacy or tolerability issues in similar proportions, but most HA patients switch to another recombinant product.
- Introducing new rFIX options may provide individuals with HB the same opportunity to switch to other recombinant therapies to achieve their treatment goals.

REFERENCES