PRESS RELEASE

Stockholm, Sweden, 26 January 2021

Favourable results for Elocta® when evaluating treatment options for haemophilia A

The international medical journal *Advances in Therapy* has recently published data evaluating treatment options for haemophilia A. The journal reported that when using individualised prophylaxis with extended half-life (EHL) products, Elocta® (rFVIIIFc) resulted in lower annual bleed rates when compared to Jivi™ (BAY 94-9027). The report was based on an indirect comparison of pivotal clinical trial data in adults.

"We are conscious of the difficulties to conduct clinical head-to-head studies in a rare disease setting such as this," said Jennifer Cain-Birkmose, Global Head of Patient Access and Community Engagement. "Through utilising the validated MAIC research method, this analysis provides important data to further understand treatment options and demonstrates Elocta's value for people living with haemophilia," she said.

Results

- Mean annualized bleeding rate (ABR) was lower in the rFVIIIFc individualised prophylaxis group versus the BAY 94-9027 pooled prophylaxis population (3.0 vs 4.9). This difference (–1.9; 95% CI –3.5, –0.4) was both clinically relevant and statistically significant (p=0.02).
- The proportion of patients with zero bleeds was numerically higher for rFVIIIFc (45.5%) than for BAY 94-9027 (38.2%), although the difference was not statistically significant (odds ratio: 1.35; 95% CI 0.77, 2.36).
- After matching, the effective sample size for A-LONG was 81 people with haemophilia A. [i]

rFVIIIFc was approved by the European Commission in November 2015 for patients with haemophilia A of all age groups. BAY 94-9027 was approved three years later for the treatment of haemophilia A in patients older than 12.

About the MAIC analysis

Matching-adjusted indirect comparison (MAIC) is a research method adopted by health technology assessment bodies around the world, including the National Institute for Care Excellence (NICE). The analysis was performed to evaluate the relative efficacy of both extended half-life therapies for the prophylactic treatment of haemophilia A.

The indirect comparison builds on data from the individualised prophylaxis arm of the A-LONG phase 3 clinical trial evaluating rFVIIIFc in 117 people with haemophilia A, and from the pooled prophylaxis population of the PROTECT VIII phase 2/3 study evaluating BAY 94-9027 in 110 people with haemophilia A**.**

Swedish Orphan Biovitrum AB (publ)
Postal address SE-112 76 Stockholm, Sweden
Phone: +46 8 697 20 00 | www.sobi.com

To adjust for cross-study differences in baseline characteristics, propensity score weighting was used to balance demographic and clinical characteristics. Outcomes assessed from the A-LONG study and the PROTECT VIII study included ABR and percentage of patients with no bleeds during the study period. As with other MAIC analyses, matching may not adjust for all confounding factors due to differences inherent in study design and entry criteria.

About haemophilia A
Haemophilia A is a rare, genetic disorder in which the ability of a person's blood to clot is impaired due to a lack of coagulation factor VIII. Haemophilia A occurs in about one in 5,000 male births annually, and more rarely in females. The World Federation of Hemophilia estimates that approximately 170,000 people are currently diagnosed with haemophilia A world-wide. People with haemophilia A experience bleeding episodes that can cause pain, irreversible joint damage and life-threatening haemorrhages.

Prophylactic injections of factor VIII can temporarily replace the clotting factor that is needed to control bleeding and prevent new bleeding episodes. The World Federation of Hemophilia (WFH) recommends prophylaxis as the optimal therapy as it can prevent bleeds and joint destruction.

About Elocta®
Elocta® (efmoroctocog alfa) is a recombinant clotting factor therapy developed for haemophilia A using Fc fusion technology (rFVIIIFc) to prolong circulation in the body. It is engineered by fusing factor VIII to the Fc portion of immunoglobulin G subclass 1, or IgG1 (a protein commonly found in the body), enabling Elocta to use a naturally occurring pathway to extend the time the therapy remains in the body (half-life). Elocta is manufactured using a human cell line in an environment free of animal and human additives.

Elocta is approved and marketed by Sobi for the treatment of haemophilia A in the EU, UK, Iceland, Norway, Liechtenstein, Switzerland, Kuwait and Saudi Arabia. It is approved and marketed as ELOCTATE® [Antihemophilic Factor (Recombinant), Fc Fusion Protein] by Sanofi in the United States, Japan and Canada. It is also approved in Australia, New Zealand, Brazil and other countries, where Sanofi has the marketing rights.

As with any factor replacement therapy, allergic-type hypersensitivity reactions and development of inhibitors may occur in the treatment of haemophilia A. Inhibitor development has been observed with Elocta, including in previously untreated patients. Note that the indication for previously untreated patients is not included in the EU Product Information for Elocta.

About Sobi™
Sobi is a specialised international biopharmaceutical company transforming the lives of people with rare diseases. Sobi is providing sustainable access to innovative therapies in the areas of haematology, immunology and specialty indications. Today, Sobi employs approximately 1,500 people across Europe, North America, the Middle East, Russia and North Africa. In 2019, Sobi's revenues amounted to SEK 14.2 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. You can find more information about Sobi at www.sobi.com.

For more information please contact
Paula Treutiger, Head of Communications & Investor Relations
+46 733 666 599
paula.treutiger@sobi.com

[i] Hakimi Z, Santagostino E, Postma MJ, Nazir J. Recombinant FVIIIFc Versus BAY 94-9027 for Treatment of Patients with Haemophilia A: Comparative Efficacy Using a Matching Adjusted Indirect Comparison. Adv Ther. 2020 Dec 30. doi: 10.1007/s12325-020-01599-1. Epub ahead of print. PMID: 33377987.