PRESS RELEASE

Stockholm, Sweden, 26 March 2021

Evaluation of haemophilia A treatment options shows favourable efficacy results for Elocta®

The *Journal of Blood Medicine* has recently published data evaluating treatment options for haemophilia A. The publication reported in an indirect comparison that individualised prophylaxis with Elocta® (rFVIIIFc-efmoroctog alfa) is more efficacious than Hemlibra™ (emicizumab) administered every four weeks, and at least as effective as more frequent emicizumab regimens for the management of haemophilia A. The report was based on a matching-adjusted indirect comparison (MAIC) of pivotal clinical trial data in adolescents and 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 at Sobi. "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[1]

- The proportion of patients with zero bleeds was significantly higher with rFVIIIFc compared with emicizumab administered every four weeks (51.2% versus 29.3%, respectively; odds ratio 2.53; 95% confidence interval 1.09–5.89); no significant differences were noted when rFVIIIFc was compared with emicizumab administered once every week or every two weeks.[2]
- After matching, no significant differences were observed between mean ABR for rFVIIIFc and emicizumab administered once every week, every two weeks or every four weeks.

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 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 HAVEN 3 and HAVEN 4 clinical trials evaluating emicizumab in 99 and 41 patients respectively. As with other MAIC analyses, matching may not adjust for all confounding factors due to differences inherent in study design and entry criteria.

The analysis excluded once-weekly arm data from A-LONG in accordance with the Elocta SmPC which doesn't include once weekly dosing as the recommended posology.

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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 2020, Sobi's revenues amounted to SEK 15.3 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. You can find more information about Sobi at www.sobi.com.

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[1]Klamroth R, Wojciechowski P, Aballéa S, Diamand F, Hakimi Z, Nazir J, Abad-Franch L, Lethagen S, Santagostino E, Tarantino MD. Efficacy of rFVIIIFc versus Emicizumab for the Treatment of Patients with Hemophilia A without Inhibitors: Matching-Adjusted Indirect Comparison of A-LONG and HAVEN Trials. *J Blood Med*. 2021;12:115-122 https://doi.org/10.2147/JBM.S288283

[2] In the individualised arm of the A-LONG study the rFVIIIFc dosing was 25–65 IU/kg every 3–5 days. The patients' pharmacokinetic (PK) parameters were used to guide individual dosing adjustments to target a steady-state FVIII trough level of 1–3 IU/dL. Emicizumab was administered as a subcutaneous injection of 3 mg/kg once weekly for the first 4 weeks (loading dose), followed by a maintenance dose of 1.5 mg/kg once weekly, 3 mg/kg every two weeks, or 6 mg/kg every four weeks.