PRESS RELEASE

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Interim data evaluating Elocta® for Immune Tolerance Induction in people with inhibitors and severe haemophilia A shared at ISTH 2019

Interim results from verITI-8, an ongoing prospective, phase 4 study evaluating the efficacy of Elocta® (efmoroctocog alfa), for first-time Immune Tolerance Induction (ITI) for people with severe haemophilia A with inhibitors were presented today. These early data from the joint Sobi™ and Sanofi study in this investigational setting were shared in an oral session at the XXVII Congress of the International Society on Thrombosis and Haemostasis (ISTH).

The development of inhibitors, or antibodies, to FVIII replacement therapy is one of the most serious complications for people with haemophilia, occurring in approximately 30 percent of people with severe haemophilia A during their lifetime. Eradication of inhibitors through ITI is currently the standard of care. ITI requires factor replacement therapy to be given regularly over a period to train the immune system to accept the treatment without reacting to it. Inhibitor eradication using conventional rFVIII has typically been seen in 12-18 months, but more difficult cases can take two years or longer. Elocta is not currently approved for ITI.

"Development of inhibitors continues to be a significant challenge for people with haemophilia and it remains an area of unmet need," says Armin Reininger, Head of Medical and Scientific Affairs at Sobi. "These early data are encouraging, and we look forward to evaluating Elocta's use in ITI for people with severe haemophilia A and inhibitors further. This study and our ongoing research together with Sanofi are part of our commitment to helping people with haemophilia liberate their lives so they can live beyond their condition."

VerITI-8 is an ongoing prospective, open label study evaluating Elocta for first time ITI in subjects with severe haemophilia A and high titre inhibitors (historical peak ≥5 BU/ml). The primary endpoint is time to tolerisation (successful ITI) with Elocta, and secondary endpoints include annualised bleed rates, number of subjects who achieve ITI success, adverse events and consumption.

In verITI-8, ITI tolerisation (success) is defined as having a negative Bethesda titre at two consecutive physician visits, normal recovery (an incremental recovery ≥66% at two consecutive physician visits) and pharmacokinetics (Elocta half-life ≥7 hours).

In this interim analysis, 15 patients with a history of high titre inhibitors and no prior ITI therapy, received ITI treatment which included administration of 200 IU/kg/day of Elocta until tolerisation or up to 48 weeks. At the data cutoff (January 23, 2019), six patients had been successfully tolerised with a median time to tolerisation of 11.7 weeks. Eight subjects in the study continue to receive Elocta ITI therapy and one has failed.

No adverse events related to rFVIIIFc have been reported. There have not been any discontinuation of study drug or early withdrawal from the study due to adverse events.

The most frequently occurring adverse reactions seen with Elocta (>0.5% of subjects) in other clinical trials were joint and muscle pain, malaise, headache, and rash.

Further data and analysis are needed to better understand and confirm these interim results. Sobi and Sanofi have a second ongoing study, ReITIrate (NCT03103542), evaluating Elocta in ITI in haemophilia A patients with inhibitors who had previously failed ITI therapies with other FVIII products.

About Elocta®

Elocta® (efmoroctocog alfa) is a recombinant clotting factor therapy developed for haemophilia A using Fc fusion technology 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, Iceland, Kuwait, Liechtenstein, Norway, Saudi Arabia and Switzerland. 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. For more information, please see the full US prescribing information for ELOCTATE. Note that the indication for previously untreated patients and ITI treatment is not included in the EU Product Information for Elocta.

About haemophilia A

Haemophilia is a rare, genetic disorder in which the ability of a person's blood to clot is impaired. Haemophilia A occurs in about one in 5,000 male births annually, and more rarely in females. The World Federation of Haemophilia estimates that approximately 158,000 people are currently diagnosed with haemophilia A and B worldwide.[i]

People with haemophilia A experience significant bleeding episodes some of which can be life-threatening. Prophylactic infusions of factor VIII or IX can temporarily replace the clotting factors that are needed to control bleeding and prevent new bleeding episodes.[ii] The World Federation of Hemophilia prophylactic factor replacement therapy for patients with haemophila to help prevent bleeding.[iii]

About the Sobi and Sanofi collaboration

Sobi and Sanofi collaborate on the development and commercialisation of Alprolix and Elocta/ELOCTATE®. Sobi has final development and commercialisation rights in the Sobi territory (essentially Europe, North Africa, Russia and most Middle Eastern markets). Sanofi has final development and commercialisation rights in North America and all other regions in the world excluding the Sobi territory and has manufacturing responsibility for Elocta/ELOCTATE and Alprolix. While Fc fusion technology has been used for more than 15 years, Sobi and Sanofi have optimised the technology and are the first companies to utilise it in the treatment of haemophilia. In 2014,

Sobi added the rFVIIIFc-XTEN-vWF fusion molecule for potential treatment of haemophilia A, and in 2017, Sobi elected to add the rFIXFc-XTEN fusion molecule for potential treatment of haemophilia B to the collaboration agreement.

About Sobi™
At Sobi, we are transforming the lives of people affected by rare diseases. As a specialised international biopharmaceutical company, we provide sustainable access to innovative therapies in the areas of haematology, immunology and specialty care. We bring something rare to rare diseases – a belief in the strength of focus, the power of agility and the potential of the people we are dedicated to serving. The hard work and dedication of our approximately 1,050 employees around the globe have been instrumental in our success across Europe, North America, the Middle East, Russia and North Africa, leading to total revenues of SEK 9.1 billion in 2018. 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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[i] The WFH Annual Global Survey 2017 https://www.wfh.org/en/data-collection
[ii] World Federation of Hemophilia. About Bleeding Disorders – Frequently Asked Questions. Available at: http://www.wfh.org/en/page.aspx?pid=637. Accessed on 4 July 2019.
[iii] World Federation of Hemophilia. Guideline for the management of hemophilia, 2nd edition. Available at: http://www1.wfh.org/publication/files/pdf-1472.pdf. Accessed on 4 July 2019.