PRESS RELEASE

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Data presented at ISTH2020 strengthens evidence for both Elocta® and Alprolix® in previously untreated patients with haemophilia

Final results from the PUPs A-LONG and PUPs B-LONG studies assessing the efficacy and safety of Elocta® (efmoroctocog alfa) and Alprolix® (eftrenonacog alfa) in previously untreated patients (PUPs) with haemophilia A and B respectively were presented at the ISTH2020 (International Society on Thrombosis and Haemostasis) Virtual Congress, July 12-14. The results were presented jointly by Sobi™ and Sanofi, collaborators in the development and commercialisation of Alprolix and Elocta/ELOCTATE.

"These are the first published final results for extended half-life (EHL) treatments in PUPs demonstrating tolerance and efficacy in this vulnerable patient population," said Milan Zdravkovic, Head of Research & Development and Chief Medical Officer at Sobi. "Each year, haemophilia A and B affect one in 5,000 and 20,000 males born[1] respectively, and this data marks a breakthrough in building evidence of efficacy and safety of Elocta and Alprolix in these patient populations. The results further strengthen the growing body of evidence generated from clinical study and post-marketing experience with Elocta and Alprolix."

PUPs A-LONG (NCT02234323) was the first study evaluating an EHL, recombinant factor VIII Fc-fusion protein (rFVIIIFc) for prevention and treatment of bleeds in PUPs with severe haemophilia A. Data demonstrates that Elocta was well tolerated and effective in this paediatric patient population. The overall development of neutralising antibodies, inhibitors, was in the expected range.

PUPs B-LONG (NCT02234310) was the first study of recombinant factor IX Fc-fusion protein (rFIXFc) for prevention and treatment of bleeds in PUPs with haemophilia B. Alprolix was well tolerated and effective for both prophylaxis and treatment of bleeding episodes. The overall inhibitor incidence was within the expected range. Importantly, only one patient developed a low-titre inhibitor, and no high-titre inhibitors were developed.

Method and endpoints

PUPs A-LONG was an open-label, multicentre, phase 3 study that enrolled male PUPs aged <6 years with haemophilia A (<1 IU/dL endogenous FVIII) to receive rFVIIIFc. The primary endpoint was

inhibitor development and secondary endpoints included annualised bleeding rate (ABR) and assessment of response to treatment of bleeding episodes with rFVIIIFc.

PUPs B-LONG was an open-label, multicentre, multinational, phase 3 study including male PUPs aged <18 years with haemophilia B (≤2 IU/dL endogenous FIX) to receive rFIXFc. The primary endpoint was occurrence of inhibitor development and secondary endpoints included annualised bleeding rate (ABR) and assessment of response to treatment of bleeding episodes with rFIXFc.

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, 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 Alprolix®

Alprolix® (eftrenonacog alfa), is a recombinant clotting factor therapy developed for haemophilia B using Fc fusion technology to prolong circulation in the body. It is engineered by fusing factor IX to the Fc portion of immunoglobulin G subclass 1, or IgG1 (a protein commonly found in the body), enabling Alprolix to use a naturally occurring pathway to extend the time the therapy remains in the body (half-life). Alprolix is manufactured using a human cell line in an environment free of animal and human additives. Alprolix is approved and marketed by Sobi for the treatment of haemophilia B in the EU, Iceland, Kuwait, Liechtenstein, Norway, Saudi Arabia and Switzerland. It is also approved in the United States, Canada, Japan, Australia, New Zealand, Brazil and other countries where Sanofi has the marketing rights.

Allergic-type hypersensitivity reactions and development of inhibitors have been observed with Alprolix in the treatment of haemophilia B, including in previously untreated patients. For more information, please see the full U.S. prescribing information for Alprolix. Note that the indication for previously-untreated patients is not included in the EU Product Information.

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 September 2019, Sobi exercised early opt-in for the development and commercialisation of BIVV001, an investigational factor VIII therapy with the potential to provide extended protection from bleeds with once-weekly dosing for people with haemophilia A.

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 1,400 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.

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1. https://www.cdc.gov/ncbddd/hemophilia/data.html