PRESS RELEASE

Stockholm, Sweden, 23 February 2023

FDA approves once-weekly efanesoctocog alfa, a new class of high-sustained factor VIII therapy for haemophilia A

Sobi® today announced that the US Food and Drug Administration (FDA) has approved efanesoctocog alfa [Antihemophilic Factor (Recombinant), Fc-VWF-XTEN Fusion Protein-ehtl], a first-in-class, high-sustained factor VIII replacement therapy for adults and children with haemophilia A. Efanesoctocog alfa is the first and only haemophilia A treatment that provides patients with normal to near-normal factor VIII activity levels for a significant part of the week with once-weekly dosing, resulting in superior protection from bleeds compared to existing factor VIII prophylaxis.

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 factor VIII. Haemophilia A occurs in about one in 5,000 male births annually, and more rarely in females. People with haemophilia can experience bleeding episodes that can cause pain, irreversible joint damage and life-threatening haemorrhages. Despite advancements in treatment made in recent years, a large unmet medical need still exists and requires further improvement in the standard of care.

"This approval marks an important clinical advancement for the haemophilia community because we have an option that can achieve higher levels of factor activity with a single weekly dose," said Lynn Malec, MD, Medical Director of Comprehensive Center for Bleeding Disorders and Associate Investigator at Versiti Blood Research Institute, and Associate Professor of Medicine and Pediatrics at The Medical College of Wisconsin, US. "By maintaining high levels of factor activity throughout the week, patients can be confident in the bleed protection efanesoctocog alfa offers."

"On behalf of Sobi I'd like to congratulate our co-development partner Sanofi on this great achievement," said Anders Ullman, Head of Research & Development and Medical Affairs, Chief Medical Officer at Sobi. "This is a major milestone in our collaboration as we work towards providing access to this important new treatment for people living with haemophilia A around the world."

The FDA approval is primarily based on data from the pivotal XTEND-1 phase 3 study recently published in The New England Journal of Medicine. Once-weekly efanesoctocog alfa met the primary endpoint, providing significant improvements in bleed protection for people with severe haemophilia A with median and mean annualised bleeding rates (ABR) of 0.00 (interquartile range: 0.00-1.04) and 0.71 (95% confidence interval: 0.52-0.97), respectively. Efanesoctocog alfa met the key secondary endpoint with a significant reduction of 77% in ABR versus prior factor prophylaxis based on an intra-patient comparison. Efanesoctocog alfa prophylaxis with 50IU/kg/week provided mean factor VIII activity greater than 40% for the majority of the week with a trough activity of 15% on day seven, and these levels were associated with a low bleed risk.

Additional data showed prevention of joint bleeds with a median annualised joint bleeding rate of 0 (Q1, Q3: 0.0, 1.0). Treatment with efanesoctocog alfa provided 100% resolution of target joints, which are joints that have recurrent bleeds (e.g., knee, ankle, or elbow). In adults and adolescents, efanesoctocog alfa had a favourable safety profile and there were no signs of factor VIII inhibitor development.

Efanesoctocog alfa is indicated in the US for routine prophylaxis, on-demand treatment and control of bleeding episodes, and perioperative management of bleeding. The recommended weekly dose of 50 IU/kg is intended for prophylaxis in patients of all ages and the same dose can be administered according to needs in different clinical settings. Efanesoctocog alfa is expected to be commercially available in the US starting in the second quarter of 2023 and will be marketed as ALTUVIIIO™ in Sanofi territories.

The FDA evaluated the application under Priority Review, which is granted to therapies that have the potential to provide significant improvements in the treatment, diagnosis, or prevention of serious conditions.

Regulatory submission in the EU will follow the availability of final data from the XTEND-Kids paediatric study in the first half of 2023. The European Commission granted orphan designation in June 2019.

About efanesoctocog alfa
Efanesoctocog alfa (formerly BIVV001) is a novel and investigational recombinant factor VIII therapy with the potential to deliver near-normal factor activity levels for most of the week, extending bleed protection in a once-weekly dose for people with haemophilia A. Efanesoctocog alfa builds on the innovative Fc fusion technology by adding a region of von Willebrand factor and XTEN® polypeptides to potentially extend its time in circulation. It is the only therapy that has been shown to break through the von Willebrand factor ceiling, which is believed to impose a half-life limitation on current factor VIII therapies. It is approved as ALTUVIIIO [Antihemophilic Factor (Recombinant), Fc-VWF-XTEN Fusion Protein-ehtl] by Sanofi in the US. It is not approved in any country outside the US.

About the Sanofi and Sobi collaboration
Sobi and Sanofi collaborate on the development and commercialisation of Alprolix® and Elocta®/Eloctate®. The companies also collaborate on the development and commercialisation of efanesoctocog alfa or ALTUVIIIO in the US. 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.

Sanofi
Sanofi are an innovative global healthcare company, driven by one purpose: to chase the miracles of science to improve people's lives. Their team, across some 100 countries, is dedicated to transforming the practice of medicine by working to turn the impossible into the possible. Sanofi provide potentially life-changing treatment options and life-saving vaccine protection to millions of people globally, while putting sustainability and social responsibility at the centre of our ambitions. Sanofi is listed on EURONEXT: SAN and NASDAQ: SNY.

Sobi®
Sobi is a specialised international biopharmaceutical company transforming the lives of people with rare and debilitating diseases. Providing reliable access to innovative medicines in the areas of haematology, immunology and specialty care, Sobi has approximately 1,600 employees across Europe, North America, the Middle East, Asia and Australia. In 2022, revenue amounted to SEK 18.8 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. More about Sobi at sobi.com, LinkedIn and YouTube.

Contacts
For details on how to contact the Sobi Investor Relations Team, please click here. For Sobi Media contacts, click here.