PRESS RELEASE

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Positive topline results from pivotal XTEND-Kids phase 3 study of efanesoctocog alfa in children under 12 years of age with haemophilia A

Primary endpoint was met with no factor VIII inhibitors detected, confirming the safety profile of efanesoctocog alfa in previously treated patients under 12 years of age

The completion of XTEND-Kids represents the final milestone needed for regulatory submission in the EU

Sobi® and Sanofi today announced that the XTEND-Kids phase 3 pivotal study evaluating the safety, efficacy and pharmacokinetics of efanesoctocog alfa as once-weekly prophylaxis in previously treated patients under 12 years of age with severe haemophilia A met its primary endpoint. No factor VIII inhibitors were observed in the 74 children enrolled in the study, of which 65 experienced at least 50 exposure days. Efanesoctocog alfa provided high sustained factor VIII levels throughout the weekly dosing interval with a median annualised bleeding rate (ABR) of 0.00 (interquartile range: 0.00-1.02) and an estimated mean ABR (95% confidence interval) of 0.89 (0.56-1.42).

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. Efanesoctocog alfa is a new class of factor VIII replacement therapy decoupled of von Willebrand factor, providing high sustained factor activity levels with a once-weekly prophylactic treatment regimen.

"These data confirm that efanesoctocog alfa has the potential to become a new standard of care for haemophilia A with higher protection for longer across treatment scenarios for all age groups," said Anders Ullman, MD, PhD, Head of Research & Development and Medical Affairs and Chief Medical Officer at Sobi. "We are excited to move ahead with regulatory submission in the EU and look forward to sharing these results at a future medical meeting."

Combined with the XTEND-1 phase 3 trial, these results will provide the basis for regulatory submission in the EU. Efanesoctocog alfa was granted orphan designation by the European Commission in June 2019 and the medicine was recently approved in the US.

About XTEND-Kids

XTEND-Kids is an open-label, non-randomised interventional, single-arm study. Participants received a weekly prophylactic dose of efanesoctocog alfa for 52 weeks. XTEND-Kids evaluates efficacy, safety and pharmacokinetics in 74 previously treated patients <12 years of age with severe haemophilia A.

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 a significant part 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] in the US.

XTEN® is a registered trademark of Amunix Pharmaceuticals, Inc.

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. 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 center 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.

This information is information that Sobi is obliged to make public pursuant to the EU Market Abuse Regulation. The information was submitted for publication, through the agency of the contact person set out below, on 2 March 2023 at 08:00 CET.

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