PRESS RELEASE

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Efanesoctocog alfa met primary and key secondary endpoints in pivotal study in haemophilia A, demonstrating superiority to prior factor prophylaxis treatment

Once-weekly efanesoctocog alfa met primary endpoint in phase 3 study, resulting in a clinically meaningful prevention of bleeding episodes (bleed protection)

In the key secondary endpoint, efanesoctocog alfa demonstrated superiority in prevention of bleeding episodes, showing a statistically significant and clinically meaningful reduction in annualised bleeding rate compared to prior factor VIII prophylaxis therapy

Efanesoctocog alfa is a novel and investigational factor VIII therapy designed to provide near-normal factor activity levels for the majority of the week in a once-weekly prophylactic treatment regimen

Swedish Orphan Biovitrum AB (publ) (Sobi®) (STO:SOBI) and Sanofi (EURONEXT: SAN and NASDAQ: SNY) today announced positive top-line results from the pivotal XTEND-1 phase 3 study evaluating the safety, efficacy and pharmacokinetics of efanesoctocog alfa (BIVV001) in previously treated patients ≥12 years of age with severe haemophilia A.

The study met the primary endpoint, showing a clinically meaningful prevention of bleeds in people with severe haemophilia A receiving weekly prophylaxis with efanesoctocog alfa over a period of 52 weeks. The median annualised bleeding rate (ABR) was 0 with a mean ABR of 0.71. The key secondary endpoint was also met, demonstrating once-weekly efanesoctocog alfa was superior to prior prophylactic factor VIII replacement therapy, showing a statistically significant reduction in ABR based on intra-patient comparison. Efanesoctocog alfa was well-tolerated, and inhibitor development to factor VIII was not detected. The most common treatment-emergent adverse events (>5 per cent of participants overall) were headache, arthralgia, fall, and back pain.

“We believe once weekly efanesoctocog alfa has the potential to represent a new class of factor VIII therapy designed to provide high sustained factor VIII activity levels near normal for the majority of the week,” said Anders Ullman, MD, PhD, Head of Research & Development and Chief Medical Officer at Sobi. “We look forward to sharing these phase 3 results, including data on physical health, pain and joint health at future medical meetings.”

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.

"While advances have been made in the treatment of haemophilia, unmet medical needs still exist. These positive top-line data, showing a very low annualised bleeding rate, enhance efanesoctocog alfa's potential to transform haemophilia A therapy. We believe efanesoctocog alfa provides higher protection for longer duration with reduced treatment burden of once-weekly dosing, and we look forward to working with regulators to bring this therapy to patients as soon as possible," said Dietmar Berger, MD, PhD, Global Head of Development at Sanofi.

The data will be the basis for submission to regulatory authorities around the globe beginning this year. Submission in the EU will follow availability of data from the ongoing XTEND-Kids paediatric study, expected in 2023. Efanesoctocog alfa was granted Orphan Drug Designation by the US Food and Drug Administration (FDA) in August 2017 and the European Commission in June 2019. The US FDA granted Fast Track Designation in February 2021. Efanesoctocog alfa is currently under clinical investigation and its safety and efficacy have not been evaluated by any regulatory authority.

About phase 3 XTEND-1 study
The phase 3 XTEND-1 study (NCT04161495) is an open-label, non-randomised interventional study assessing the safety, efficacy and pharmacokinetics of efanesoctocog alfa in people 12 years of age or older (n=159) with severe haemophilia A who were previously treated with factor VIII replacement therapy. The study includes two parallel treatment arms – the prophylaxis arm, where study participants received a weekly prophylactic 50 IU/kg dose of efanesoctocog alfa for 52 weeks (Arm A), some of which were enrolled following an observation period on prophylaxis using currently marketed factor VIII replacement therapies, and an on-demand arm, where study participants received 50 IU/kg as needed for 26 weeks followed by weekly prophylaxis for another 26 weeks (Arm B).

The primary efficacy endpoint was the annualised bleeding rate (ABR) in arm A, and the key secondary endpoint was an intra-patient comparison of ABR during the efanesoctocog alfa weekly prophylaxis treatment period versus the prior prophylaxis ABR for participants in arm A who participated in study 242HA201/OBS16221, an observational study.

About efanesoctocog alfa (BIVV001)
Efanesoctocog alfa is a novel and investigational recombinant factor VIII therapy that is designed to extend protection from bleeds with once-weekly prophylactic dosing for people with haemophilia A. It builds on the innovative Fc fusion technology by adding a region of von Willebrand factor and XTEN® [1] polypeptides to extend its time in circulation. It is the first investigational factor VIII therapy that has been shown to break through the von Willebrand factor ceiling, which imposes a half-life limitation on current factor VIII therapies. Efanesoctocog alfa is currently under clinical investigation and its safety and efficacy have not been evaluated by any regulatory authority.

About the Sobi and Sanofi 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, an investigational factor VIII therapy with the potential to provide high sustained factor activity levels with once-weekly dosing for people with haemophilia A. 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.

[1] XTEN® is a registered trademark of Amunix Pharmaceuticals, Inc

Sanofi
Sanofi is 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 provides 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 their 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 diseases. Providing sustainable 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 and Asia. In 2021, revenue amounted to SEK 15.5 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. More about Sobi at sobi.com, LinkedIn and YouTube.

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 persons set out below, at 07:00 CET on 9 March 2022.

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