PRESS RELEASE

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FDA grants efanesoctocog alfa Breakthrough Therapy designation for haemophilia A

Efanesoctocog alfa is the first factor VIII therapy to be awarded Breakthrough Therapy designation by the FDA

Designation is based on XTEND-1 phase 3 study data demonstrating a clinically meaningful prevention of bleeds and superiority in prevention of bleeding episodes compared to prior prophylaxis factor treatment

Efanesoctocog alfa is a novel and investigational factor VIII therapy designed to provide normal to 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 that the United States Food and Drug Administration (FDA) has granted Breakthrough Therapy designation to efanesoctocog alfa (BIVV001) for the treatment of people with haemophilia A, a rare and life-threatening bleeding disorder, based on data from the pivotal XTEND-1 phase 3 study. Sanofi and Sobi collaborate on the development and commercialisation of efanesoctocog alfa.

Breakthrough Therapy designation is designed to expedite the development and review of drugs in the US that target serious or life-threatening conditions. Drugs qualifying for this designation must show preliminary clinical evidence that the drug may demonstrate a substantial improvement on clinically significant endpoints over available therapies.

“The Breakthrough Therapy designation highlights efanesoctocog alfa’s potential to transform treatment for people with haemophilia A by providing higher protection for longer duration,” said John Reed, MD, PhD, Global Head of Research and Development at Sanofi. “This potential new class of factor VIII therapy represents how we are boldly advancing science to address unmet needs for the haemophilia community. We are excited to work with regulatory authorities during the filing and review of this innovative therapy.”

“This designation supports the innovation of efanesoctocog alfa and acknowledges its potential to fulfil an unmet medical need for people living with haemophilia A,” said Anders Ullman, MD, PhD, Head of Research & Development and Chief Medical Officer at Sobi. “We are committed to transforming lives for people living with rare diseases, and this is a testament to the medical innovation that science can bring.”

Topline results from the pivotal XTEND-1 phase 3 study demonstrated that efanesoctocog alfa met the primary endpoint, showing a clinically meaningful prevention of bleeds in people with severe haemophilia A over a 52-week period. Importantly, the key secondary endpoint was also met, demonstrating that efanesoctocog alfa was superior to prior prophylactic factor VIII replacement therapy in preventing bleeding events based on an 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.

Data from the XTEND-1 phase 3 study are expected to be shared at an upcoming medical meeting and will serve as the basis for regulatory submission to the FDA by mid-year 2022. The FDA granted efanesoctocog alfa Orphan Drug designation in August 2017 and Fast Track designation in February 2021. The European Commission also granted efanesoctocog alfa Orphan Drug designation in June 2019. Regulatory submission in the EU will follow availability of data from the ongoing XTEND-Kids paediatric study, expected in 2023.

Haemophilia A occurs in about one in 5,000 male births annually, and more rarely in females. It is a lifelong condition in which the ability of a person's blood to clot is impaired due to a coagulation factor deficiency. People with haemophilia can experience bleeding episodes that can cause pain, irreversible joint damage, and life-threatening haemorrhages. Unmet medical needs remain for people with haemophilia to strengthen protection, reduce treatment burden and improve quality of life.

About efanesoctocog alfa (BIVV001)
Efanesoctocog alfa is a novel and investigational recombinant factor VIII (FVIII) 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® polypeptides to extend its time in circulation. It is the first investigational FVIII therapy that has been shown to break through the von Willebrand factor ceiling, which imposes a half-life limitation on current FVIII 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.

About Sanofi
We are an innovative global healthcare company, driven by one purpose: we chase the miracles of science to improve people's lives. Our team, across some 100 countries, is dedicated to transforming the practice of medicine by working to turn the impossible into the possible. We 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 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.

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