PRESS RELEASE

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FDA grants priority review to efanesoctocog alfa for people with haemophilia A

The FDA decision date for efanesoctocog alfa, an investigational factor VIII therapy, is set for 28 February 2023

Priority review is based on pivotal data from the XTEND-1 phase 3 study

Efanesoctocog alfa delivers high sustained factor activity levels in the normal to near-normal range for the majority of the week with once weekly prophylaxis dosing, providing higher protection for longer

The US Food and Drug Administration (FDA) has accepted for priority review the Biologics License Application (BLA) for efanesoctocog alfa (BIVV001) for the treatment of haemophilia A, a rare and life-threatening bleeding disorder. The target action date for the FDA decision is 28 February 2023. Sobi® and Sanofi collaborate on the development of efanesoctocog alfa.

“Factor therapy remains a cornerstone of haemophilia treatment, but innovation has been needed in this area to address challenges related to bleed protection and cumbersome treatment regimens,” said Steve Pipe, MD, Professor and Director of Paediatric Haemophilia and Coagulation Disorders Program, University of Michigan. “If approved, efanesoctocog alfa can deliver close to normal factor activity levels for the majority of the week, potentially offering a new tier of protection. Such therapeutic benefits would represent important advances in unmet medical needs for people with haemophilia A and may transform the prophylactic treatment landscape.”

The BLA is supported by data from the pivotal XTEND-1 phase 3 study. Results were recently presented at the 30th International Society of Thrombosis and Haemostasis Congress. The data demonstrate a clinically meaningful prevention of bleeds and superiority to prior factor prophylaxis 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% of participants overall) were headache, arthralgia, fall, and back pain.

"We believe transforming the treatment paradigm for haemophilia A can only be achieved through elevating standards of care towards normal haemostasis," said Anders Ullman, MD, PhD, Head of Research & Development and Chief Medical Officer at Sobi. “Today’s news further acknowledges the potential of efanesoctocog alfa to ultimately improve the lives of many people living with this condition.”

The FDA grants priority review to therapies that have the potential to provide significant improvements in the treatment, diagnosis, or prevention of serious conditions. Efanesoctocog alfa received Breakthrough Therapy designation from the FDA in May 2022 and it is the first factor VIII therapy to receive this recognition. The FDA also granted efanesoctocog alfa Orphan Drug designation in August 2017 and Fast Track designation in February 2021.

Regulatory submission in the EU will follow availability of data from the ongoing XTEND-Kids paediatric study, with both events expected in 2023. The European Commission granted efanesoctocog alfa Orphan Drug designation in June 2019.

About haemophilia A

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.

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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. Factor replacement therapy remains a cornerstone of care and can be used across multiple treatment scenarios.

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® 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 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.

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 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 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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