PRESS RELEASE

Stockholm, Sweden, 26 April 2024

Sobi® receives positive CHMP opinion recommending approval of efanesoctocog alfa for once-weekly treatment of haemophilia A

Sobi® today announced the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency has issued a positive opinion recommending approval of efanesoctocog alfa, for the treatment and prevention of bleeds and perioperative prophylaxis in haemophilia A. Efanesoctocog alfa is a once-weekly and high-sustained factor VIII replacement therapy for patients of all ages and any disease severity.

Efanesoctocog alfa provides children, adolescents and adults with normal to near-normal factor VIII activity levels (above 40%) for a significant part of the week with once-weekly dosing, reaching trough levels of 15% in adults and adolescents at day 7. This results in significantly improved protection from bleeds compared to existing factor VIII prophylaxis. The CHMP positive opinion will now be submitted to the European Commission for a marketing authorisation decision.

“Today’s announcement marks a major milestone in haemophilia care and moves us one step closer to bringing efanesoctocog alfa to patients in the EU. Efanesoctocog alfa sustains high factor VIII activity levels throughout the week, giving patients confidence in the protection it can provide to prevent bleeds, manage surgery, and resolve bleeds. With the potential to significantly improve treatment outcomes and quality of life for people living with haemophilia A, we are excited about the positive impact this treatment could have around the world,” said Lydia Abad-Franch, MBA, Head of Research, Development, and Medical Affairs, and Chief Medical Officer at Sobi.

Haemophilia A is a rare, lifelong genetic condition in which the body does not produce enough, or makes dysfunctional, factor VIII – a protein that is essential for blood clotting. It 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. Clinical outcomes have improved over time thanks to significant advances in the treatment options available, however important unmet clinical and social needs still exist for those living with the condition.

The recommendation from the CHMP is based on the results from the pivotal phase 3 studies: XTEND-1 in adults and adolescents and XTEND-Kids in children, which evaluated the efficacy and safety of efanesoctocog alfa. These studies demonstrated that once-weekly efanesoctocog alfa prophylaxis (50 IU/kg) provided significant bleed protection (mean ABR <1 and 80-88% of patients free from spontaneous bleeds) in people with severe haemophilia A of any age. The data also showed substantial improvement in joint health, physical health, pain and overall quality of life when comparing week 52 and baseline measurements.[1] No factor VIII inhibitors were observed in the efanesoctocog alfa clinical program.

Efanesoctocog alfa was first approved in the US in February 2023 by the US Food and Drug Administration (FDA). The FDA previously granted efanesoctocog alfa Breakthrough Therapy designation in May 2022 — the first factor VIII therapy to receive this designation, Fast Track designation in February 2021, and Orphan Drug designation in 2017.

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References

1. von Drygalski. A., Chowdary. P., Kulkarni. R., et al. Efanesoctocog Alfa Prophylaxis for Patients with Severe Haemophilia A. N Engl J Med 2023; 388:310-318.

About XTEND-1

XTEND-1 was an open-label, non-randomized interventional study with two parallel assignment arms. Participants in the prophylaxis arm received a weekly prophylactic dose of efanesoctocog alfa for 52 weeks. Participants in on-demand arm received efanesoctocog alfa on demand for 26 weeks followed by a switch to weekly prophylaxis for another 26 weeks. XTEND-1 evaluated efficacy, safety and pharmacokinetics in 159 previously treated patients ≥12 years of age with severe haemophilia A.

About XTEND-Kids

XTEND-Kids was 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 [Antihemophilic Factor (Recombinant), Fc-VWF-XTEN Fusion Protein] (formerly BIVV001) is a new class of recombinant factor VIII therapy with the potential to deliver near-normal factor activity levels for a significant part of the week, improving 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 extend its time in circulation. It is the only therapy that has been shown to break through the von Willebrand factor ceiling, which imposes a half-life limitation on current factor VIII therapies. It is approved and marketed as ALTUVIIIO™ [Antihemophilic Factor (Recombinant), Fc-VWF-XTEN Fusion Protein-ehtl] by Sanofi in the United States, Japan, and Taiwan. The European Commission granted Orphan Drug designation in June 2019.

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.

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 and debilitating diseases. Providing reliable access to innovative medicines in the areas of haematology, immunology, and specialty care, Sobi has approximately 1,800 employees across Europe, North America, the Middle East, Asia, and Australia. In 2023, revenue amounted to SEK 22.1 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. More about Sobi at sobi.com and LinkedIn.

Contacts

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