PRESS RELEASE

Stockholm, Sweden, 19 June 2024

European Commission grants Sobi® Marketing Authorisation for ALTUVOCT™ for treatment of haemophilia A

Sobi® today announced the European Commission has granted Marketing Authorisation for ALTUVOCT™ (efanesoctocog alfa), for the treatment and prevention of bleeds and perioperative prophylaxis in haemophilia A. ALTUVOCT is a high-sustained factor VIII replacement therapy for all ages and any disease severity. Children, adolescents, and adults can experience non-haemophilia factor VIII activity levels (above 40%) for a significant part of the week with once-weekly prophylaxis, reaching trough levels of 15% in adults and adolescents before the next dose. This results in significantly improved protection from bleeds compared to prior factor VIII prophylaxis.

The European Commission also endorsed the European Medicines Agency (EMA) recommendation supporting ALTUVOCT's retention of orphan drug designation, granting a 10-year market exclusivity period. The EMA recommendation noted that even considering existing treatments, once-weekly ALTUVOCT prophylaxis demonstrated a significantly lowered annual bleeding rate compared to other factor VIII products, and this constitutes a clinically relevant advantage.

“Despite advancements, haemophilia still limits the possibilities of patients’ lives and this means there is still a need for treatments that offer elevated protection. ALTUVOCT’s high-sustained factor VIII activity and once-weekly dosing schedule have the potential to significantly improve quality of life for people with haemophilia A. The trials demonstrated substantial improvements in the prevention and treatment of bleeds along with significant improvements in physical health, pain, and joint health,” said Professor Robert Klamroth, MD, PhD, Head of the Department of Internal Medicine, Vascular Medicine and Coagulation Disorders at the Vivantes Klinikum Friedrichshain, Berlin, Germany.

The granting of Marketing Authorisation 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 ALTUVOCT in people with severe haemophilia A. These studies demonstrated that once-weekly ALTUVOCT prophylaxis (50 IU/kg) provided significant bleed protection for any age (mean ABR <1 and 80-88% of patients free from spontaneous bleeds). The outcomes also showed substantial improvement in joint health, physical health, pain and overall quality of life when comparing week 52 and baseline assessments.[1,2] No factor VIII inhibitors were observed in the ALTUVOCT clinical program.

“Today’s announcement marks a major step forward in haemophilia care, offering the potential to significantly improve treatment outcomes and quality of life. For the first time, factor VIII activity levels can be sustained for a significant part of the week with simplified once-weekly dosing. We are proud to work alongside the haemophilia community, as we lead the paradigm shift towards normal haemostasis and create new possibilities together,” 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.

ALTUVOCT 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.

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.
2. Malec, L., Dunn, A., Carcao, M., Zulfikar, B., Berrueco, R., Brown, S., Khan, U., Gunawardena, S., Neill, G., Abad-Franch, L., Bystrická, L., Santagostino, E., & Susen, S. (2023). Treatment of Bleeding Episodes with Efanesoctocog Alfa in Children with Severe Hemophilia A in the XTEND-Kids Phase 3 Study. Blood, 142 (Supplement 1), 3993.

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 ALTUVOCT™

ALTUVOCT (efanesoctocog alfa) [Antihemophilic Factor (Recombinant), Fc-VWF-XTEN Fusion Protein] (formerly BIVV001) is the first high-sustained FVIII replacement 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 established Fc fusion technology by innovatively 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. The European Commission granted Orphan Drug designation in June 2019 It is approved and marketed as ALTUVOCT™ by Sobi in Europe. 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.

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 ALTUVOCT™, 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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