PRESS RELEASE

Stockholm, Sweden, 25 June 2023

Late-breaking efanesoctocog alfa data presented at ISTH demonstrates highly effective bleed protection in children with severe haemophilia A with once-weekly dosing

XTEND-Kids data confirms the efficacy and safety profile of efanesoctocog alfa with 50 IU/kg dosing in previously treated children as already shown in adults and adolescents. Factor VIII inhibitor development was not detected during the study.

Once-weekly efanesoctocog alfa was shown to sustain FVIII levels in the normal near-normal range (above 40%) for approximately 3 days in patients under 12 years of age, providing effective bleed protection throughout the weekly dosing interval.

Pivotal data from the Phase 3 XTEND-Kids study evaluating efanesoctocog alfa [Antihemophilic Factor (Recombinant), Fc-VWF-XTEN Fusion Protein] once-weekly prophylaxis in previously treated patients younger than 12 years of age with severe haemophilia A were presented today in a late-breaking session at the Annual Meeting of the International Society on Thrombosis and Haemostasis (ISTH) in Montreal, Canada.

The oral presentation detailed results from the XTEND-Kids study and confirmed that efanesoctocog alfa met the primary endpoint, occurrence of inhibitor development, and secondary endpoints including annualised bleeding rate (ABR) In the paediatric population, clearance of administered factor concentrates in the blood is greater than in adults, often requiring multiple injections per week using SHL or EHL factor VIII products. These data confirm that a once-weekly 50 IU/kg dose of efanesoctocog alfa provides highly effective bleed protection in both children and adults and can be used across all clinical scenarios.

“Haemophilia A is often diagnosed at a very young age. The requirement for haemophilia patients to be careful in their physical activities to prevent bleeding episodes and joint injuries is especially challenging for young children. They also need frequent infusions of prophylactic treatments. The results from XTEND-Kids demonstrate that efanesoctocog alfa offers highly effective bleed protection also for paediatric patients, with a once-weekly dosing. This is encouraging, as it highlights its potential to become a new standard of care for haemophilia A in patients of all age groups,” said Lydia Abad-Franch, MD, PhD, Head of Research & Development and Medical Affairs and Chief Medical Officer at Sobi.

Key Results

The Phase 3 XTEND-Kids study (NCT04759131) was an open-label, non-randomised interventional study of the safety, efficacy, and pharmacokinetics of once-weekly efanesoctocog alfa in previously treated patients younger than 12 years of age with severe haemophilia A. Patients (n=74) received once-weekly efanesoctocog alfa prophylaxis (50 IU/kg) for a mean duration of 51 weeks.

- Development of Factor VIII inhibitors was not detected (0% [95% confidence interval (CI)] 0–4.9]).
- Efanesoctocog alfa was well-tolerated and demonstrated a safety profile consistent with the XTEND-1 trial, confirming safety in both adults and children.
- No serious allergic reactions, anaphylaxis, or embolic or thrombotic events were reported. No adverse events led to treatment discontinuation.

- Median (interquartile range) and mean ABRs (95% CI) were 0.00 (0.00–1.02) and 0.89 (0.56–1.42), respectively.
- 64% of patients had zero bleeding episodes, 82% of patients had zero joint bleeds and 88% of patients had zero spontaneous bleeds.
- Pharmacokinetic results showed that weekly efanesoctocog alfa prophylaxis provided FVIII levels above 40% for approximately 3 days and above 10% at approximately day 7

Haemophilia A is a rare, lifelong condition in which the ability of a person's blood to clot properly is impaired, leading to excessive and spontaneous bleeds into joints that can result arthropathy and chronic pain, and potentially impact quality of life. The severity of haemophilia is determined by the level of clotting factor activity in a person's blood, and there is a negative correlation between risk of bleeding and factor activity levels.

Efanesoctocog alfa, commercialised as ALTUVIIIO™ in the United States, is a new class of high-sustained factor VIII (FVIII) replacement therapy approved in February 2023 by the US Food and Drug Administration (FDA) for routine prophylaxis and on-demand treatment to control of bleeding episodes, as well as perioperative management (surgery) for adults and children with haemophilia A. Granted Breakthrough Therapy designation by the FDA in May 2022 – the first FVIII therapy to receive this designation—ALTUVIIIO also received Fast Track designation in February 2021 and Orphan Drug designation in 2017. The European Commission granted Orphan Drug designation in June 2019, and the European Medicines Agency accepted the Marketing Authorization Application (MAA) for efanesoctocog alfa in May 2023.

About XTEND-Kids
The XTEND-Kids study (NCT04759131) was an open-label, non-randomized interventional study of the safety, efficacy, and pharmacokinetics of once-weekly ALTUVIIIO in previously treated patients younger than 12 years of age with severe hemophilia A. Patients received once-weekly ALTUVIIIO prophylaxis (50 IU/kg) for 52 weeks which provided high-sustained FVIII levels throughout the weekly dosing interval with a median (IQR) annualized bleeding rate (ABR) of 0.00 (0.00, 1.02) and an estimated mean (95% CI) ABR of 0.89 (0.56 ; 1.42). The primary endpoint was the occurrence of inhibitor development (baseline to 52 weeks). No inhibitors were detected in this study.

About the XTEND Clinical Programs
The XTEND clinical program is comprised of two Phase 3 trials in hemophilia A: XTEND-1 in people 12 years or older and XTEND-Kids in children younger than 12 years old. There is also an ongoing extension study (XTEND-ed).

The Phase 3 XTEND-1 study (NCT04161495) was an open-label, non-randomized interventional study assessing the safety, efficacy, and pharmacokinetics of once-weekly ALTUVIIIO in people 12 years of age or older (n=159) with severe hemophilia A who were previously treated with factor VIII replacement therapy. The study consisted of two parallel treatment arms — the prophylaxis Arm A (n=133), in which patients who had received prior factor VIII prophylaxis were treated with once-weekly intravenous ALTUVIIIO prophylaxis (50 IU/kg) for 52 weeks, and the on-demand Arm B (n=26), in which patients who had received prior on-demand factor VIII therapy began with 26 weeks of on-demand ALTUVIIIO (50 IU/kg), then switched to once-weekly prophylaxis with ALTUVIIIO (50 IU/kg) for an additional 26 weeks.

The primary efficacy endpoint of XTEND-1 was the mean annualized bleeding rate (ABR) in Arm A, and the key secondary endpoint was an intra-patient comparison of ABR during the ALTUVIIIO weekly prophylaxis treatment period versus the prior factor VIII prophylaxis ABR for a subset of participants in Arm A who had participated in a previous observational study (Study 242HA201/OBS16221).

The XTEND-Kids study (NCT04759131) was an open-label, non-randomized interventional study of the safety, efficacy, and pharmacokinetics of once-weekly ALTUVIIIO in previously treated patients younger than 12 years of age with severe hemophilia A. Patients received once-weekly ALTUVIIIO prophylaxis (50 IU/kg) for 52 weeks. The primary endpoint was the occurrence of inhibitor development.

About the Sanofi and Sobi collaboration
Sobi and Sanofi collaborate on the development and commercialization of Alprolix® and Elocta®/Eloctate®. The companies also collaborate on the development and commercialization of efanesoctocog alfa, or ALTUVIIIO in the US. Sobi has final development and commercialization rights in the Sobi territory (essentially Europe, North Africa, Russia and most Middle Eastern markets). Sanofi has final development and commercialization 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

About 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 1,600 employees across Europe, North America, the Middle East, Asia and Australia. In 2022, revenue amounted to SEK 18.8 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. More about Sobi www.sobi.com, LinkedIn and YouTube.

Contacts
For details on how to contact the Sobi Investor Relations Team, please click here. For Sobi Media contacts, click here.