PRESS RELEASE

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NEJM publishes data demonstrating clinically meaningful prevention of bleeds and superior bleed protection for efanesoctocog alfa

Pivotal study data published in The New England Journal of Medicine (NEJM) continues to highlight the efficacy, safety, and pharmacokinetic profile of efanesoctocog alfa (formerly BIVV001), a potential new medicine for haemophilia A. These data demonstrate the clinical benefits of normal to near-normal factor activity levels (>40%) for the majority of the week achieved by once weekly efanesoctocog alfa dosing. Efanesoctocog alfa is currently under priority regulatory review in the US with a target action date of 28 February 2023.

Haemophilia A is a rare, lifelong condition in which the ability of a person's blood to clot properly is impaired, leading to excessive bleeds that can result in joint damage 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.

"We are excited about the potential for efanesoctocog alfa to address unmet needs by allowing people living with haemophilia to enjoy an active lifestyle. Currently, they often need to make trade-offs between bleed protection and dosing frequency," said Angela Weyand MD, Investigator of the XTEND-1 clinical study and Associate Professor at Michigan Medicine. "Based on the XTEND-1 study results assessing efanesoctocog alfa, we have the opportunity to provide near-normal factor activity levels for an extended period of time (the majority of a week) with a single dose, which is a first for haemophilia A. The data show that efanesoctocog alfa can offer patients increased bleed protection, leading to improved outcomes, such as reduced pain and improved physical functioning, that may impact daily life with a reduced treatment burden."

The data from the pivotal XTEND-1 phase 3 study results published in NEJM show that efanesoctocog alfa met primary and key secondary endpoints, demonstrating clinically meaningful prevention of bleeds and superior bleed protection compared to prior prophylaxis based on an intra-patient comparison. Treatment with efanesoctocog alfa prophylaxis also resulted in significant and clinically meaningful improvements in physical health, pain, and joint health. Key results include:

- The median and mean annualised bleeding rates (ABR) were 0.00 (interquartile range: 0.00-1.04) and 0.71 (95% CI: 0.52-0.97), respectively.
- A statistically significant and clinically meaningful reduction in ABR (77%) versus prior factor VIII prophylaxis ($p<0.001$).
- 97% of bleeding episodes resolved with a single injection of efanesoctocog alfa (50 IU/kg).
- Efanesoctocog alfa provided mean factor activity >40 IU/dL for the majority of the week and at 15 IU/dL on day 7.
- Efanesoctocog alfa prophylaxis improved physical health ($p<0.001$), pain intensity ($p=0.03$), and joint health ($p=0.01$) when comparing 52 week and baseline measurements.[1]
- In patients with target joints at baseline, 100% of the target joints were resolved after at least 12 months of continuous prophylaxis.
- 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, Head of Research &

Development and Medical Affairs, Chief Medical Officer at Sobi. "The publication of the X-TEND-1 study phase 3 results by NEJM reflect the significance of these data for medical and haemophilia patient communities around the world."

About XTEND-1
The XTEND-1 phase 3 study (NCT04161495) was an open-label, non-randomised interventional study assessing the safety, efficacy and pharmacokinetics of once-weekly efanesoctocog alfa in people 12 years of age or older (n=159) with severe haemophilia 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 efanesoctocog alfa 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 26 weeks of on-demand efanesoctocog alfa (50 IU/kg), then switched to once-weekly prophylaxis (50 IU/kg) for an additional 26 weeks.

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

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 missing or defective factor VIII clotting protein. 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
Efanesoctocog alfa (formerly BIVV001) is a novel and investigational recombinant factor VIII therapy with the potential to deliver near-normal factor activity levels for most of the week, extending 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 potentially extend its time in circulation. It is the only therapy that has been shown to break through the von Willebrand factor ceiling, which is believed to impose a half-life limitation on current factor VIII therapies.

Efanesoctocog alfa is currently under clinical investigation and its safety and efficacy have not been reviewed by any regulatory authority. Efanesoctocog alfa was granted Orphan Drug Designation by the US Food & Drug Administration (FDA) in August 2017 and the European Commission in June 2019. The FDA has accepted for priority review the Biologics License Application for efanesoctocog alfa for the treatment of haemophilia A. The target action date for the FDA decision is 28 February 2023.

Regulatory submission in the EU, anticipated in the second half of 2023, will follow availability of data from the ongoing, fully recruited XTEND-Kids paediatric study, expected in the first half of 2023. The European Commission granted efanesoctocog alfa orphan designation in June 2019. Sanofi and Sobi collaborate on the development of efanesoctocog alfa.

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.

Reference
1. Physical health was assessed with the Haem-A-QoL Physical Health score. Pain intensity was assessed using the PROMIS Pain Intensity 3a past 7 days intensity of pain at its worst score.

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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