PRESS RELEASE

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Pivotal data demonstrate once-weekly efanesoctocog alfa provides superior bleed protection compared to prior factor prophylaxis

Investigational once-weekly efanesoctocog alfa prophylaxis met the primary efficacy endpoint providing clinically meaningful bleed protection for people with severe haemophilia A

Results underscore the ability of efanesoctocog alfa to sustain normal to near-normal factor levels and the potential to transform prophylactic treatment, providing people with haemophilia A with higher protection for longer

Additional data showed efanesoctocog alfa prophylaxis resulted in statistically significant and clinically meaningful improvements in physical health, pain intensity and joint health in patients on prior factor VIII prophylaxis

Swedish Orphan Biovitrum AB (publ) (Sobi®) (STO:SOBI) and Sanofi (EURONEXT:SAN and NASDAQ:SNY) presented for the first time today, in a late-breaking session at the 30th International Society on Thrombosis and Haemostasis (ISTH) Congress, positive results from the XTEND-1 pivotal phase 3 study evaluating the safety, efficacy and pharmacokinetics of efanesoctocog alfa (BIVV001), an investigational factor VIII replacement therapy, in previously treated adults and adolescents ≥12 years with severe haemophilia A.

The study met the primary efficacy endpoint, with once-weekly efanesoctocog alfa prophylaxis providing clinically meaningful bleed protection for people with severe haemophilia A. The median and mean annualised bleeding rates (ABR) were 0.00 (IQR: 0.00 – 1.04) and 0.71 (SD: 1.43) respectively. The study also met the key secondary endpoint, demonstrating superior bleed protection ($p<0.0001$) over prior factor VIII prophylaxis with an estimated ABR reduction of 77 per cent and a mean ABR of 0.69 compared to 2.96 on prior prophylaxis, based on an intra-patient comparison (n=78). In a subset of participants (n=17) studied at baseline and week 26, mean factor VIII levels remained in the normal to near-normal range (>40 IU/dL) for the majority of the week, and at 15 IU/dL at day seven post dosed, providing increased factor activity level protection for patients with once-weekly prophylaxis.

Annette von Drygalski, MD, PharmD, Investigator, Professor and Director, Hemophilia and Thrombosis Treatment Center, UC San Diego said; “The phase 3 data demonstrate once-weekly efanesoctocog alfa’s potential to provide superior bleed protection, leading to substantial improvements in physical health, pain and joint health, by sustaining high factor levels for the majority of the week. These unprecedented results may offer people with haemophilia A the possibility to redefine their treatment expectations.”

Data show adults and adolescents treated with once-weekly efanesoctocog alfa experienced statistically significant and clinically meaningful improvements in physical health ($p=0.0001$), pain intensity ($p=0.0276$), and joint health ($p=0.0101$) when comparing week 52 and baseline measurements[1]. Moreover, efanesoctocog alfa was effective at treating bleeds, including in target joints; 96.7 per cent of bleeds were resolved with a single 50 IU/kg dose. Efanesoctocog alfa was well tolerated and inhibitor development to factor VIII was not detected. The most common treatment-emergent adverse events (>5 per cent 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. “These data demonstrate the profile of efanesoctocog alfa in significant clinical terms, and further strengthen its potential to ultimately improve the lives of many living with this condition.”

“We are committed to advancing innovative medicines that disrupt the status-quo and address the unmet needs that persist for people with rare conditions like haemophilia. These robust data illustrate the promise of efanesoctocog alfa’s efficacy with once-weekly dosing and underscore its potential as a therapy with best-in-disease efficacy”, said Dietmar Berger, MD, PhD, Global Head of Development and Chief Medical Officer at Sanofi.

The US Food and Drug Administration (FDA) granted efanesoctocog alfa Breakthrough Therapy Designation in May 2022, Fast Track designation in February 2021 and Orphan Drug designation in August 2017. The European Commission also granted efanesoctocog alfa Orphan Drug designation in June 2019. Regulatory submission of the Biologics License Application to the US FDA occurred in June 2022 and submission in the EU will follow availability of data from the ongoing XTEND-Kids paediatric study, expected in 2023.

About the XTEND-1 phase 3 study
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 consists of two parallel treatments arms — the prophylaxis Arm A (n=133), in which patients who had received prior factor VIII prophylaxis began receiving 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 rates (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 lack of factor VIII. 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 (FVIII) 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 FVIII therapy that has been shown to break through the von Willebrand factor ceiling, which imposes a half-life limitation on current FVIII therapies. Efanesoctocog alfa is currently under clinical investigation and its safety and efficacy have not been evaluated by any regulatory authority.

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 efanesoctocog alfa, an investigational factor VIII therapy with the potential to provide high sustained factor activity levels with once-weekly dosing for people with haemophilia A. 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 center of our ambitions. Sanofi is listed on EURONEXT: SAN and NASDAQ: SNY

References

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. Joint health was assessed using the Hemophilia Joint Health 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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