PRESS RELEASE

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First patient dosed in phase 3 XTEND-Kids study with efanesoctocog alfa in children with haemophilia A

Swedish Orphan Biovitrum AB (publ) (Sobi™) today announced that the first patient has been dosed in the phase 3, open-label, interventional XTEND-Kids study of efanesoctocog alfa (BIVV001) in paediatric subjects with severe haemophilia A. The XTEND-Kids study is designed to investigate the efficacy, safety and pharmacokinetics of efanesoctocog alfa as once-weekly prophylaxis in approximtately 65 previously treated patients ≤12 years of age. Duration of treatment in the study is 52 weeks. Efanesoctocog alfa is being developed and will be commericalized in partnership with Sanofi.

Factor VIII (FVIII) replacement therapy as a single agent has been shown in clinical studies and real-world experience to control and prevent bleeding effectively in a variety of settings including surgery[1]. Efanesoctocog alfa represents a potential new class of FVIII replacement therapy with high sustained factor levels, and the first recombinant FVIII therapy that is designed to be von Willebrand factor (VWF) independent, which extends half-life. It is currently in clinical development and has the potential to offer extended protection with once weekly dosing[2], thus addressing an unmet need for people living with severe haemophilia A.

“Efanesoctocog alfa has the potential to advance the treatment of people with haemophilia A” says Ravi Rao, Head of R&D and Chief Medical Officer at Sobi. “The initiation of the phase 3 development in the paediatric population is a demonstration of Sobi’s continued commitment to enable higher standards for people living with haemophilia A”.

Efanesoctocog alfa is currently in phase 3 clinical trials and was granted Fast Track designation by the US Food and Drug Administration (FDA) in February 2021. It was also granted Orphan Drug designation by both the US FDA in August 2017 and the European Commission in June 2019.

XTEND-Kids

XTEND-Kids is an open-label, non-randomised interventional, single-arm study. Participants will receive a weekly prophylactic dose of efanesoctocog alfa for 52 weeks. XTEND-Kids will evaluate efficacy, safety and pharmacokinetics in approximately 65 previously treated patients ≤12 years of age with severe haemophilia A.

About efanesoctocog alfa (BIVV001)

Efanesoctocog alfa (rFVIIIFc-VWF-XTEN) is a novel and investigational recombinant factor VIII therapy that is designed to extend protection from bleeds with once-weekly prophylactic dosing 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 was granted orphan drug designation by the US Food & Drug Administration in August 2017 and the European Commission in June 2019. Efanesoctogoc alfa is currently under clinical investigation and its safety and efficacy have not been reviewed by any regulatory authority.

About the Sobi and Sanofi collaboration

Sobi and Sanofi collaborate on the development and commercialisation of Alprolix® and Elocta®/ELOCTATE®. 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 and has manufacturing responsibility for Elocta/ELOCTATE and Alprolix. While Fc fusion technology has been used for more than 15 years, Sobi and Sanofi have optimised the technology and are the first companies to utilise it in the treatment of haemophilia. Since September 2019, Sobi and Sanofi collaborate in the development and commercialisation of efanesoctocog alfa (BIVV001), an investigational factor VIII therapy with the potential to provide extended protection from bleeds with once-weekly dosing for people with haemophilia A.

About Sobi™

Sobi is a specialised international biopharmaceutical company transforming the lives of people with rare diseases. Sobi is providing sustainable access to innovative therapies in the areas of haematology, immunology and specialty indications. Today, Sobi employs approximately 1,500 people across Europe, North America, Middle East and Asia. In 2020, Sobi's revenues amounted to SEK 15.3 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. You can find more information about Sobi at sobi.com.

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References:

1. Srivastava, A., Brewer, A.K., Mauser-Bunschoten, E.P., Key, N.S., Kitchen, S., Llinas, A., Ludlam, C.A., Mahlangu, J.N., Mulder, K., Poon, M.C., Street, A. and (2013), Guidelines for the management of hemophilia. Haemophilia, 19: e1-e47.
2. Konkle BA, Shapiro AD, Quon DV, Staber JM, Kulkarni R, Ragni MV, Chhabra ES, Poloskey S, Rice K, Katragadda S, Fruebis J, Benson CC. BIVV001 Fusion Protein as Factor VIII Replacement Therapy for Hemophilia A. N Engl J Med. 2020 Sep 10;383(11):1018-1027.