PRESS RELEASE

Stockholm, Sweden, 11 December 2019

First patient dosed in BIVV001 phase 3 XTEND-1 study in people with severe haemophilia A

Sobi™ and Sanofi today announced that the first patient has been dosed in the phase 3, open-label, interventional study of BIVV001 (rFVIIIFc-VWF-XTEN), in patients with severe haemophilia A (XTEND-1 study; NCT04161495). Sobi and Sanofi are development partners for BIVV001 and the XTEND-1 study is designed to investigate the efficacy, safety and tolerability of prophylactic once-weekly dosing.

Factor VIII (FVIII) replacement therapy is a single therapy that can be used across treatment scenarios including, prophylaxis, acute bleed control and perioperative haemostasis. FVIII replacement products that offer extended bleed protection with once-weekly dosing intervals remain an unmet need for people living with severe haemophilia A.

Final results from the phase 1 repeat dosing study of BIVV001 were recently presented at the 61st Annual Meeting of the American Society of Hematology (ASH) in December 2019, Orlando, FL US. The study evaluated the safety and pharmacokinetics of repeat dosing of BIVV001 and showed the potential to provide high sustained levels of factor VIII (FVIII) activity with once-weekly dosing. The study also indicated that BIVV001 was safe and well tolerated.

“Based on the final data of the phase 1 repeat dose study, we are pleased to initiate phase 3 development. BIVV001 has the potential to further advance the treatment of people with haemophilia A also by normalising FVIII levels for part of the treatment interval, and we look forward to moving this important development program forward,” says Milan Zdravkovic, Head of R&D and Chief Medical Officer at Sobi.

BIVV001 was awarded Orphan Drug designation by the U.S. Food and Drug Administration and European Commission. This designation provides incentives to advance the development and commercialisation of rare disease therapies.

“We’re pleased to have enrolled the first patient in this phase 3 clinical trial and advance the BIVV001 program,” said Karin Knobe, MD, PhD, Therapeutic Head of Development, Rare Blood Disorders, Sanofi. “Based on the promising results from the phase 1 trial evaluating BIVV001, we look forward to understanding the full clinical potential of this investigational factor replacement therapy with a hope that it continues to demonstrate high sustained factor levels with longer prophylactic dosing intervals, an area of unmet need for patients.”

BIVV001 is a novel and investigational recombinant factor VIII therapy that builds on the Fc fusion technology by adding a region of von Willebrand factor and XTEN® polypeptides to extend its time in circulation. BIVV001 is the first von Willebrand (VWF)-independent factor VIII therapy designed to provide high sustained factor activity and extend protection from bleeds with once-weekly dosing for people with haemophilia A.

Study design

XTEND-1 is an open-label, non-randomized interventional study with two parallel assignment arms. Participants in the prophylaxis arm will receive a weekly prophylactic dose of BIVV001 for 52 weeks. Participants in on-demand arm will receive BIVV001 on demand for 26 weeks followed by a switch to weekly prophylaxis for another 26 weeks. XTEND-1 will evaluate efficacy, safety and pharmacokinetics in approximately 150 previously treated patients ≥12 years of age with severe haemophilia A.

About BIVV001

BIVV001 (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. BIVV001 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. BIVV001 was granted orphan drug designation by the US Food and Drug Administration in August 2017 and the European Commission in June 2019.

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. In September 2019, Sobi exercised early opt-in for the development and commercialisation of 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

At Sobi, we are transforming the lives of people affected by rare diseases. As a specialised international biopharmaceutical company, we provide sustainable access to innovative therapies in the areas of haematology, immunology and specialty care. We bring something rare to rare diseases – a belief in the strength of focus, the power of agility and the potential of the people we are dedicated to serving. The hard work and dedication of our approximately 1,300 employees around the globe has been instrumental in our success across Europe, North America, the Middle East, Russia and North Africa, leading to total revenues of SEK 9.1 billion in 2018. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. You can find more information about Sobi at www.sobi.com.

About Sanofi

Sanofi is dedicated to supporting people through their health challenges. We are a global biopharmaceutical company focused on human health. We prevent illness with vaccines, provide innovative treatments to fight pain and ease suffering. We stand by the few who suffer from rare diseases and the millions with long-term chronic conditions.

With more than 100,000 people in 100 countries, Sanofi is transforming scientific innovation into healthcare solutions around the globe.

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