

PRESS RELEASE

Stockholm, Sweden, 10 September 2020

***New England Journal of Medicine* publishes positive final results from phase 1/2a study of BIVV001 in people with severe haemophilia A**

- BIVV001 is the first investigational factor VIII therapy independent of von Willebrand factor and has the potential to transform replacement therapy for people with haemophilia A
- It is uniquely designed to deliver near-normal factor activity levels for the majority of the week, extending bleed protection in a once-weekly dose
- Results from the phase 1/2a study showed that a single dose of BIVV001 achieved high sustained factor activity and a three- to four-fold increase in half-life when compared to conventional factor VIII replacement therapies

Stockholm & Paris – The [*New England Journal of Medicine*](#) today published positive final results from the phase 1/2a trial evaluating the safety, tolerability and pharmacokinetics of BIVV001 (rFVIII_{Fc}-VWF-XTEN) in adult patients with severe haemophilia A. BIVV001 is an investigational factor VIII therapy designed to provide higher bleed protection in a once-weekly prophylactic treatment regimen. Sobi™ (STO:SOBI) and Sanofi collaborate on the development and commercialization of BIVV001.

“BIVV001 represents a potential new class of factor VIII replacement therapies. The sustained factor activity levels and three- to four-fold increase in half-life observed underscore its potential to provide near-normal bleed protection while reducing the dosing frequency of a prophylactic treatment to once a week,” said Barbara A. Konkle, MD, lead investigator and Chief Scientific Officer, Bloodworks Northwest and Professor of Medicine/Haematology, University of Washington. “These results support the conclusion that BIVV001 may be a significant advancement for patients and we look forward to exploring this further in the ongoing phase 3 study.”

Phase 1/2a study results

EXTEN-A is an open-label, multicentre study that evaluated the safety, tolerability and pharmacokinetics of BIVV001 in both a 25 IU/kg (n=6) and a 65 IU/kg (n=8) dose cohort of participants aged 19-63 years with severe haemophilia A (NCT03205163). In the trial, participants received a single dose of conventional recombinant factor VIII (rFVIII) followed, after a washout period, by either a single 25 IU/kg or 65 IU/kg dose of BIVV001. Primary endpoints included occurrence of adverse events and development of inhibitors. Key findings included:

- BIVV001 was generally well tolerated with no inhibitor development detected through 28 days post-dose. During the study period no adverse events of allergic reaction, anaphylaxis or clinically meaningful treatment-related adverse events were reported.

- In the 65 IU/kg dose cohort, a single dose of BIVV001 achieved a FVIII half-life of 43 hours, a greater than three-fold increase from the 13-hour half-life observed with rFVIII. Mean factor VIII activity level was $\geq 51\%$ and in the normal range for four days, and 17% at seven days post BIVV001 infusion.
- In the 25 IU/kg cohort, a single dose of BIVV001 achieved a FVIII half-life of 38 hours, a four-fold increase from the 9-hour half-life observed with rFVIII, with a mean factor activity level of 5% at seven days post BIVV001 infusion.

Factor activity levels refer to the amount of factor VIII in a person's blood and are used to determine the severity of a person's disease. Participants enrolled in the EXTEN-A trial have severe haemophilia A (factor levels of $<1\%$). Moderate haemophilia A is characterized by factor levels of 1-5%, and mild haemophilia A is from 5-40%.

A potential to transform factor replacement therapy for haemophilia A

The half-life of conventional factor VIII therapy is constrained by the von Willebrand factor's (VWF) chaperone effect, which is believed to limit the time the factor remains in the body. BIVV001 is the first factor VIII therapy under development that has been shown to break through the VWF ceiling, thus allowing people with haemophilia A to potentially move toward normal factor activity levels for the majority of the week.

"As part of our overall commitment to the haemophilia community, we are excited by the clinical potential of BIVV001 to overcome the limitations of current factor VIII therapies," said Dietmar Berger, Global Head of Development and Chief Medical Officer at Sanofi. "The New England Journal of Medicine's publication of these early results support the possibility of BIVV001 to provide people with haemophilia A with higher protection for longer, which could allow them to lead a more active life. We look forward to providing future updates as we continue to evaluate BIVV001 in phase 3 development."

"Factor VIII replacement therapy remains a cornerstone of care in haemophilia A and is a single therapy that can be used across numerous treatment scenarios including, prophylaxis, acute bleed control and perioperative management," said Ravi Rao, Head of R&D and Chief Medical Officer at Sobi. BIVV001 has the potential to advance factor replacement therapy further by offering patients and physicians near-normal factor levels for the majority of the week whilst reducing treatment burden. We look forward to exploring this further in the phase 3 study."

Phase 3 XTEND-1 study

The safety and efficacy of BIVV001 is currently being evaluated in the ongoing phase 3 XTEND-1 study in previously treated patients ≥ 12 years of age (n=150) with severe haemophilia A. 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 50 IU/kg dose of BIVV001 for 52 weeks. Participants in the on-demand arm

will receive BIVV001 (50 IU/kg) on demand for 26 weeks followed by a switch to BIVV001 weekly prophylaxis for another 26 weeks.

About BIVV001

BIVV001 (rFVIIIIFc-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 hemophilia A. BIVV001 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 factor VIII therapy that has been shown to break through the von Willebrand factor ceiling, which imposes 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. BIVV001 is currently under clinical investigation and its safety and efficacy have not been reviewed by any regulatory authority.

About Sanofi

Sanofi is dedicated to supporting people through their health challenges. Sanofi is a global biopharmaceutical company focused on human health. Sanofi prevents illness with vaccines, provides innovative treatments to fight pain and ease suffering. Sanofi stands 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.

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 1,400 people across Europe, North America, the Middle East, Russia and North Africa. In 2019, Sobi's revenues amounted to SEK 14.2 billion. Sobi's share (STO:SObi) is listed on Nasdaq Stockholm. You can find more information about Sobi at www.sobi.com.

For more information please contact

Paula Treutiger, Head of Communication & Investor Relations
+46 733 666 599
paula.treutiger@sobi.com

Linda Holmström, Corporate Communications & Investor Relations
+46 708 734 095
linda.holmstrom@sobi.com

Sanofi Media Relations Contact

Sally Bain
Tel.: +1 (781) 264 1097
Sally.Bain@sanofi.com

Sanofi Investor Relations Contact

Eva Schaefer-Jansen
Tel.: +33 (0)1 53 77 45 45
ir@sanofi.com