

## PRESS RELEASE

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### Sobi to present data at the ISTH 2021 within rare haematology diseases

[Swedish Orphan Biovitrum AB \(publ\)](#) (Sobi™) presents data at the virtual ISTH 2021, the 29th Congress of the International Society on Thrombosis and Haemostasis, 17 – 21 July 2021. Nine abstracts featuring four treatments have been accepted for presentation during ISTH, reflecting Sobi's commitment to the rare disease community within haematology. ISTH gather the world's leading experts to present recent advances, exchange science and discuss clinical applications designed to improve patient care within haematology.

“With the goal of advancing care and providing innovative treatment approaches for people living with rare haematologic diseases, we are excited to share data at this year’s virtual ISTH,” said Ravi Rao, Head of R&D and Chief Medical Officer at Sobi. “These data exemplify our efforts to increase knowledge and understanding of rare diseases.”

#### Real-world outcomes to be presented for Alprolix® and Elocta®

Data will be presented on Alprolix (eftrenonacog alfa) and Elocta (efmoroctocog alfa), both extended half-life factor therapies for people with haemophilia A and B. Sobi collaborates with Sanofi on the development and commercialization of Elocta and Alprolix.

#### Oral presentation

- First Interim Analysis of a 24-month French, Multicentre, Prospective, Non-interventional Study Evaluating the Real-world Usage and Effectiveness of the Extended Half-life Recombinant Factor IX Fc Fusion Protein (rFIXFc) in People with Haemophilia B (B-SURE). Session: Hemophilia: Real World Data. Sunday, July 18 (11:00-12:00 EST). # OC 08.1.

#### ePosters

- Interim Analysis of the PREVENT Study: Real World Prospective Data from Children and Adolescents with Haemophilia A or B Treated with Recombinant Factor VIII Fc (rFVIII Fc) or Recombinant Factor IX Fc (rFIXFc) Poster #PB0506.
- Final Results of ReITrate - A Prospective Study of Rescue Immune Tolerance Induction (ITI) with Recombinant Factor VIII Fc (rFVIII Fc) in Patients who Have Failed Previous ITI Attempts. Poster #PB0522. Joint with Sanofi.
- Real-world Usage of rFIXFc in Sweden: A Report from the Swedish National Registry for Bleedings Disorders. Poster #PB0551.

- B-MORE, Baseline Analysis from a 24-month Prospective, Non-interventional, Multicentre Study on Real-world Effectiveness and Usage of Recombinant Factor IX Fc (rFIXFc) in Haemophilia B. Poster #PB0552.

### **Real-world data for Doptelet® in the treatment of chronic immune thrombocytopenia**

Sobi will present three abstracts on the use of Doptelet (avatrombopag) in chronic immune thrombocytopenia (ITP), including real-world data from the United States.

#### *Oral presentation*

- Durability of Platelet Count Response in Patients Treated with Avatrombopag for Immune Thrombocytopenia (ITP): Post-hoc Results from the Phase 3 Core and Open-label Extension Study. Wednesday, July 21 (13:00-14:00 EST). Poster #OC 72.4.

#### *ePosters*

- Length of Thrombopoietin Receptor Agonist (TPO-RA) Treatment and Persistence in Immune Thrombocytopenia (ITP): Real World United States Claims Analyses. Poster #PB0819.
- Switching from Eltrombopag or Romiplostim to Avatrombopag in Immune Thrombocytopenia: A Multicenter Study of U.S. ITP Referral Centers. Poster #PB0823.

### **Effect on fatigue in patients with paroxysmal nocturnal haemoglobinuria (PNH)**

Data will be presented on the effect of treatment with pegcetacoplan on fatigue in patients on pegcetacoplan monotherapy and patients switching from eculizumab to pegcetacoplan.

#### *live e-poster*

- Clinically Meaningful and Long-Term Improvements in Fatigue With the C3 Inhibitor Pegcetacoplan In Paroxysmal Nocturnal Hemoglobinuria: Post-Hoc Analyses from the PEGASUS Study Week 48. Tuesday, July 20 (15:00-16:00 EST). Poster #LPB0118.

*All ePosters will be made available virtually on Saturday, July 17.*

#### **About Alprolix®**

Alprolix® (eftrenonacog alfa), is a recombinant clotting factor therapy developed for haemophilia B using Fc fusion technology to prolong circulation in the body. It is engineered by fusing factor IX to the Fc portion of immunoglobulin G subclass 1, or IgG1 (a protein commonly found in the body), enabling Alprolix to use a naturally occurring pathway to extend the time the therapy remains in the body (half-life). Alprolix is manufactured using a human cell line in an environment free of animal and human additives. Alprolix is approved and marketed by Sobi for the treatment of haemophilia B in the EU, Iceland, Kuwait, Liechtenstein, Norway,

Saudi Arabia and Switzerland. It is also approved in the United States, Canada, Japan, Australia, New Zealand, Brazil and other countries where Sanofi has the marketing rights.

#### **About Elocta®**

Elocta® (efmoroctocog alfa) is a recombinant clotting factor therapy developed for haemophilia A using Fc fusion technology (rFVIII-Fc) to prolong circulation in the body. It is engineered by fusing factor VIII to the Fc portion of immunoglobulin G subclass 1, or IgG1 (a protein commonly found in the body), enabling Elocta to use a naturally occurring pathway to extend the time the therapy remains in the body (half-life). Elocta is manufactured using a human cell line in an environment free of animal and human additives. Elocta is approved and marketed by Sobi for the treatment of haemophilia A in the EU, UK, Iceland, Norway, Liechtenstein, Switzerland, Kuwait and Saudi Arabia. It is approved and marketed as ELOCTATE® [Antihemophilic Factor (Recombinant), Fc Fusion Protein] by Sanofi in the United States, Japan and Canada. It is also approved in Australia, New Zealand, Brazil and other countries, where Sanofi has the marketing rights.

#### **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 Doptelet® (avatrombopag)**

Doptelet is an orally administered thrombopoietin receptor agonist (TPO-RA) that mimics the biologic effects of TPO in stimulating the development and maturation of megakaryocytes, resulting in increased platelet count. It is approved by the European Medicines Agency (EMA) and the US Food & Drug Administration (FDA) for the treatment of thrombocytopenia in adult patients with chronic liver disease who are scheduled to undergo a procedure, and for the treatment of thrombocytopenia in adult patients with chronic immune thrombocytopenia (ITP) who have had an insufficient response to a previous treatment. Chronic ITP is a rare autoimmune bleeding disorder characterised by low number of platelets. The incidence of primary ITP in adults is 3.3/100 000 adults per year with a prevalence of 9.5 per 100 000 adults<sup>1</sup>.

#### **About pegcetacoplan**

Pegcetacoplan is a therapy targeting C3, the central protein in the complement cascade. It acts proximally in the complement cascade controlling both C3b mediated extravascular haemolysis and terminal complement mediated intravascular haemolysis. Pegcetacoplan is being evaluated in several clinical studies across haematology, ophthalmology, nephrology, and neurology. In May 2021, pegcetacoplan was approved as EMPAVELI™ in the US for the treatment of adults with paroxysmal nocturnal haemoglobinuria (PNH). The marketing authorisation application for pegcetacoplan for PNH is under review by the European Medicines Agency (EMA). Pegcetacoplan was also granted Fast Track designation by the US Food & Drug Administration (FDA) for the treatment of geographic atrophy and received orphan drug designation for the treatment of C3 glomerulopathy by the FDA and EMA. For additional information regarding pegcetacoplan clinical studies, visit [apellis.com/our-science/clinical-trials](http://apellis.com/our-science/clinical-trials).

#### **About the Sobi and Apellis Collaboration**

Sobi and Apellis entered a collaboration to develop and commercialise systemic pegcetacoplan in October 2020. The companies have global co-development rights for systemic pegcetacoplan. Sobi has exclusive ex-US commercialisation rights for systemic pegcetacoplan, while Apellis has exclusive US commercialisation rights for systemic pegcetacoplan and retains worldwide commercial rights for ophthalmological pegcetacoplan, including for geographic atrophy (GA).

**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](https://www.sobi.com).

**For more information, please contact**

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1 (Lambert et al. Blood 2017)