

PRESS RELEASE

Stockholm, Sweden, 6 November 2020

Strong Sobi presence at American Society of Hematology 2020 Annual Meeting

Sobi™ will present data at the 62nd Annual Meeting of the American Society of Hematology (ASH) taking place as a virtual meeting from 5-8 December. Twelve abstracts featuring five treatments have been accepted for presentation during ASH, reflecting Sobi's commitment to the rare disease community.

Final data in previously untreated patients with haemophilia

Final data strengthening evidence for the efficacy and safety of Elocta® (efmoroctocog alfa) and Alprolix® (eftrenonacog alfa) in previously untreated patients (PUPs) with haemophilia A and B respectively will be presented in collaboration with Sanofi.

Oral presentation

- #509 Final results of the PUPs A-LONG Study: Evaluating Safety and Efficacy of rFVIII Fc in Previously Untreated Patients with Haemophilia A. Session: 322. 7:30 AM, Monday, 7 December 2020.

Poster abstract presentation

- #867 Final Results of PUPs B-LONG Study: Evaluating Safety and Efficacy of rFIX Fc in Previously Untreated Patients with Haemophilia B. Session: 322. Saturday, 5 December 2020.

Phase 3 study design for new class of FVIII therapy

An overview of the ongoing BIVV001 phase 3 study design (the XTEND-1 study) will be shared in a poster presentation. BIVV001 is an investigational once-weekly factor therapy for people with haemophilia A and represents a new class of FVIII therapy that has the potential to provide high sustained factor activity levels. BIVV001 is being developed in collaboration with Sanofi.

Poster abstract presentation

- #856 Evaluating BIVV001, a New Class of Factor VIII Replacement Therapy: A Phase 3 Study (XTEND-1) Design. Session: 321. Saturday, 5 December 2020.

Data for the treatment of Chronic Immune Thrombocytopenia (ITP)

Efficacy and safety data for Doptelet® (avatrombopag) in treatment for Chronic Immune Thrombocytopenia (ITP) will also be presented.

Poster abstract presentations

- #848 Pharmacokinetic/Pharmacodynamic (PK/PD) Modeling Providing Guidance for Selecting Avatrombopag (AVA) Dose When Switching from Eltrombopag in Chronic Immune Thrombocytopenia (ITP). Session: 311. Saturday, 5 December 2020.

- #835 Consistent Efficacy Demonstrated by Avatrombopag in Immune Thrombocytopenia (ITP) Regardless of the Number of Lines of Prior ITP Treatment. Session: 311. Saturday, 5 December 2020.
- #844 Characterization of Thromboembolic Events Occurring During the Avatrombopag Immune Thrombocytopenia (ITP) Clinical Development Program. Session: 311. Saturday, 5 December 2020.
- #2660 A Phase 3 Randomized, Double-Blind, Placebo-Controlled Study Evaluating the Efficacy and Safety of Avatrombopag for the Treatment of Chemotherapy-Induced Thrombocytopenia in Patients with Solid Tumors. Session: 311. Monday, 7 December 2020.
- #2675 Durability of Initial Platelet Count Response in Patients Treated with Avatrombopag for Immune Thrombocytopenia (ITP): Post-hoc Results from a Phase 3 Clinical Study. Session: 311. Monday, 7 December 2020.
- #2677 Efficacy Analyses from the Immune Thrombocytopenia (ITP) Clinical Development Program for Avatrombopag: Comparisons with Placebo and Eltrombopag. Session: 311. Monday, 7 December 2020.

Data on emapalumab to be showcased in three presentations

Key results from the pivotal phase 2/3 study will be presented, with analyses confirming the study's primary endpoint and supporting a flexible dosing scheme with a favourable safety profile for emapalumab in patients with primary haemophagocytic lymphohistiocytosis.

Poster abstract presentations

- #3266 Sensitivity Analysis of Overall Response Rate (ORR) with Emapalumab in Children with Primary Hemophagocytic Lymphohistiocytosis (HLH). Session: 704. Monday, 7 December 2020.
- #3273 Population Pharmacokinetic Analysis of Emapalumab, a Fully Human, Anti-Interferon Gamma Monoclonal Antibody, in Children with Primary Hemophagocytic Lymphohistiocytosis. Session: 704. Monday, 7 December 2020.
- #3278 Safety of Emapalumab in Children with Primary Hemophagocytic Lymphohistiocytosis: Results of the Primary Analysis of the Pivotal Phase 2/3 Study. Session: 704. Monday, 7 December 2020.

Apellis to present new data supporting the efficacy and safety of pegcetacoplan in PNH

Sobi collaboration partner Apellis will also be presenting 8 abstracts which include data supporting the efficacy and safety of pegcetacoplan, a targeted C3 therapy, for the treatment of paroxysmal nocturnal hemoglobinuria (PNH). For more information visit investors.apellis.com/news-releases.

All abstracts can be accessed via the [official ASH website](#).

About Elocta®

Elocta® (efmoroctocog alfa) is a recombinant clotting factor therapy developed for haemophilia A using Fc fusion technology 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, 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.

As with any factor replacement therapy, allergic-type hypersensitivity reactions and development of inhibitors may occur in the treatment of haemophilia A. Inhibitor development has been observed with Elocta, including in previously untreated patients. Note that the indication for previously untreated patients is not included in the EU Product Information for Elocta.

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.

Allergic-type hypersensitivity reactions and development of inhibitors have been observed with Alprolix in the treatment of haemophilia B, including in previously-untreated patients. For more information, please see the full [U.S. prescribing information](#) for Alprolix.

About BIVV001

BIVV001 (rFVIII-Fc-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 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 high sustained factor activity levels with once-weekly dosing for people with haemophilia A.

About Doptelet®

Doptelet® is an oral thrombopoietin (TPO) receptor agonist administered with food. Doptelet is approved by both the United States Food and Drug Administration (FDA) and European Medicines Agency (EMA) for treatment of thrombocytopenia (low platelet counts) in adult patients with chronic liver disease (CLD) who are scheduled to undergo a procedure. In June 2019, Doptelet was approved for the treatment of thrombocytopenia in adult patients with chronic immune thrombocytopenia (ITP) who have had an insufficient response to a previous treatment by FDA. Chronic ITP is a

rare autoimmune bleeding disorder characterised by low number of platelets, affecting approximately 60,000 adults in the United States.

About emapalumab

Emapalumab is a monoclonal antibody that binds to and neutralises interferon gamma (IFN γ). In the US, emapalumab is indicated for the treatment of adult and paediatric (new-born and older) patients with primary hemophagocytic lymphohistiocytosis (HLH) with refractory, recurrent or progressive disease or intolerance with conventional HLH therapy. Primary HLH is a rare syndrome of hyperinflammation that usually occurs within the first year of life and can rapidly become fatal unless diagnosed and treated. The FDA approval is based on data from the phase 2/3 studies (NCT01818492 and NCT02069899). Emapalumab is indicated for administration through intravenous infusion over one hour twice per week until haematopoietic stem cell transplantation (HSCT). For more information please see www.gamifant.com including the full US Prescribing Information. Emapalumab is under review for primary HLH by the European Medicines Agency (EMA). In September 2020, emapalumab received Orphan Drug Designation (ODD) by the FDA for prevention of graft failure following haematopoietic stem cell transplantation.

About Apellis

Apellis Pharmaceuticals, Inc. is a global biopharmaceutical company that is committed to leveraging courageous science, creativity, and compassion to deliver life-changing therapies. Leaders in targeted C3 therapies, we aim to develop transformative therapies for a broad range of debilitating diseases that are driven by excessive activation of the complement cascade, including those within haematology, ophthalmology, nephrology, and neurology. For more information, please visit www.apellis.com.

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, the Middle East, Russia and North Africa. In 2019, Sobi's revenue 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.

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