PRESS RELEASE

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Sobi to present new data at the ASH 2021 Annual Meeting

Sobi™ will present new data at the 63rd Annual Meeting of the American Society of Hematology (ASH) taking place from 11-14 December 2021. Sobi's commitment to developing innovative treatments for people living with rare diseases is highlighted in studies spanning several rare disorders, including haemophilia, paroxysmal nocturnal haemoglobinuria (PNH), chronic immune thrombocytopenia (ITP), macrophage activation syndrome (MAS) and haemophagocytic lymphohistiocytosis (HLH).

For the first time, the PRINCE phase 3 data on pegcetacoplan in adults with PNH who are treatment naïve – meaning they had not received a complement inhibitor within three months before entering the study – will be presented.

"We look forward to presenting data from the PRINCE study for the first time, evaluating the efficacy and safety of pegcetacoplan in people living with PNH who are treatment naïve," said Ravi Rao, Head of Research & Development and Chief Medical Officer at Sobi. "We are also excited to be able to collaborate and connect in person at this year's annual meeting."

In addition to the PRINCE data, a new post-hoc analysis on pegcetacoplan in patients who were either treatment naïve or still experiencing anaemia on treatment with the C5 inhibitor eculizumab will be presented.

In haemophilia A, new data on efanesoctocog alfa, previously known as BIVV001, will be presented. Efanesoctocog alfa, an investigational once-weekly factor therapy for people with haemophilia A, has the potential to provide high sustained factor VIII activity and near-normal factor levels for the majority of the week. Efanesoctocog alfa represents a potential new class of factor replacement treatment. Currently in phase 3 development in partnership with Sanofi, the new data from a post-hoc analysis of phase 1/2 studies will add more clarity on the new mechanism of half-life extension of efanesoctocog alfa by evaluating the independency of its pharmacokinetics from von Willebrand factor.

Sobi will also present new data from the pilot study of Gamifant® (emapalumab), a fully human, anti-interferon gamma (IFNγ) monoclonal antibody, in patients with systemic juvenile idiopathic arthritis (sJIA) developing MAS. These data demonstrate the pathogenic role of IFNγ in MAS/sJIA and the therapeutic value of IFNγ neutralisation in patients with MAS who have failed standard therapy with high-dose glucocorticoids. Additionally, new exposure-safety analyses of data obtained from the pivotal trial of Gamifant in patients with primary HLH will be presented.

Key Sobi data to be presented at ASH 2021

Haemophilia		
Alprolix® (eftrenonacog alfa)	Prophylaxis with rFIXFc Reduces the Frequency and Delays Time to First Spontaneous Bleed Event in Previously Untreated Patients with Hemophilia B: A Post Hoc Analysis of the PUPs B-LONG Trial.	#498. Oral presentation Session: 322. Sunday, 12 December, Session Time: 4:30 PM - 6:00 PM/Presentation Time: 5:45 PM ET. Joint with Sanofi.
Efanesoctocog alfa (investigational)	Efanesoctocog Alfa Half-Life and Clearance Are Independent of von Willebrand Factor in Severe Hemophilia A: A Post Hoc Analysis from Phase 1/2a Studies.	#1035. Poster presentation Saturday, 11 December, 5:30 PM - 7:30 PM. Joint with Sanofi.
Elocta®/Eloctate® (efmoroctocog alfa)	Retrospective Observational Descriptive Study on the Effectiveness and Usage of Emicizumab and Antihemophilic Factor (recombinant), Fc Fusion Protein in Patients with Hemophilia A in the US.	#3031. Poster presentation Session: 904. Sunday, 12 December, 6:00 PM - 8:00 PM ET. Presented by Sanofi.
Paroxysmal nocturnal haemoglobinuria		
Aspaveli®/Empaveli™ (pegcetacoplan)	Efficacy and Safety of Pegcetacoplan Treatment in Complement-Inhibitor Naïve Patients with Paroxysmal Nocturnal Hemoglobinuria: Results from the Phase 3 PRINCE Study.	#606. Oral presentation Monday, 13 December, 11:45 AM ET, Georgia World Congress Center, Georgia Ballroom 1-3 Joint with Apellis.
	Changes in Hemoglobin Measures Observed in PNH Patients Treated with Both C5 Inhibitors Ravulizumab and Eculizumab: Real-World Evidence from a US-Based EMR Network.	#1112. Poster presentation Saturday, 11 December, 5:30 PM-7:30 PM ET. Joint with Apellis.
	Post Hoc Analysis of the Effect of Pegcetacoplan Treatment of Patients with Paroxysmal Nocturnal Hemoglobinuria and Baseline Hemoglobin Levels Greater Than 10 Grams per Deciliter.	#2194. Poster presentation Sunday, 12 December, 6:00 PM-8:00 PM ET. Joint with Apellis.
	Evaluation of the Long-Term Safety and Efficacy of Pegcetacoplan Treatment for Paroxysmal Nocturnal Hemoglobinuria Patients: An Extension Study.	#2175. Poster presentation Sunday, 12 December, 6:00 PM-8:00 PM ET. Joint with Apellis.
	Categorized Hematologic Response to Pegcetacoplan and Correlations with Quality of Life in Patients with Paroxysmal Nocturnal Hemoglobinuria: Post Hoc Analysis of Data from Phase 1b, Phase 2a, and Phase 3 Trials.	#1104. Poster presentation Saturday, 11 December, 5:30 PM-7:30 PM ET. Presented by Apellis.
Chronic immune thrombocytopenia		
Doptelet® (avatrombopag)	Durability of Platelet Response When Switching from Eltrombopag or Romiplostim to Avatrombopag in Immune Thrombocytopenia (ITP): A Multicenter Study.	#1015. Poster presentation Session: 311. Saturday, 11 December, 5:30 PM - 7:30 PM ET.
	Further Characterization of Thromboembolic Events and Association with Platelet Count Occurring during the Avatrombopag Immune Thrombocytopenia (ITP) Clinical Development Program.	#2086. Poster presentation Session: 311. Sunday, 12 December, 6:00 PM - 8:00 PM ET.

Hemophagocytic lymphohistiocytosis		
Gamifant® (emapalumab)	Safety of Emapalumab in Pediatric Patients with Primary Hemophagocytic Lymphohistiocytosis (HLH): Relationship to Treatment Exposure.	#2061. Poster presentation. Session: 201 Sunday, 12 December, 6:00 PM - 8:00 PM ET.
Macrophage activation syndrome		
Gamifant® (emapalumab)	Macrophage Activation Syndrome (MAS) in Systemic Juvenile Idiopathic Arthritis (sJIA): Treatment with Emapalumab, an Anti-Interferon Gamma (IFNγ) Monoclonal Antibody.	#2058. Poster presentation. Session: 201 Sunday, 12 December, 6:00 PM - 8:00 PM ET.

All abstracts can be accessed via the official ASH website.

About Elocta®/Eloctate®
Elocta®/Eloctate® (efmoroctocog alfa) is a recombinant clotting factor therapy developed for haemophilia A using Fc fusion technology (rFVIIIFc) 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/Eloctate to use a naturally occurring pathway to extend the time the therapy remains in the body (half-life). Elocta/Eloctate is approved and marketed by Sobi for the treatment of haemophilia A in the EU including Russia, 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 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). Sobi and Sanofi collaborate on the development and commercialisation of Alprolix. 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 efanesoctocog alfa (BIVV001)
Efanesoctocog alfa (rFVIIIFc-VWF-XTEN) is a novel and investigational recombinant factor VIII therapy with the potential to deliver near-normal factor activity levels for most of the week, extending bleed protection in a once-weekly dose 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. Efanesoctocog alfa is currently under clinical investigation and its safety and efficacy have not been reviewed by any regulatory authority.

About Aspaveli®/Empaveli™
Aspaveli®/Empaveli™ (pegcetacoplan) is a targeted C3 therapy designed to regulate excessive activation of the complement cascade, part of the body's immune system, which can lead to the onset and progression of many serious diseases. Empaveli is approved in the United States for the treatment of adults with paroxysmal nocturnal haemoglobinuria (PNH). The Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency has adopted a positive opinion for Aspaveli for the treatment of adults with PNH who are anaemic after treatment with a C5 inhibitor for at least three months. The positive opinion from the CHMP is now referred to the European Commission for an approval decision. The therapy is also under investigation for several other rare diseases across haematology, nephrology, and neurology.

About the PRINCE study
The PRINCE study (NCT04085601) is a 2:1 (pegcetacoplan: standard of care) randomised, multi-centre, open-label, controlled phase 3 study in 53 treatment-naïve adults with paroxysmal nocturnal haemoglobinuria (PNH). The primary

objective of this study was to establish the efficacy and safety of pegcetacoplan in patients who have not received treatment with any complement inhibitor within three months prior to screening. During the 26-week randomised, controlled period, patients received either 1080 mg of pegcetacoplan twice weekly or standard of care therapy, which did not include complement inhibitors. Patients in the standard of care group had the option to escape to the pegcetacoplan group if their haemoglobin decreased 2 g/dL or more from their baseline value.

About the Sobi and Apellis Collaboration
Sobi and Apellis collaborate to develop and commercialize systemic pegcetacoplan. Sobi has exclusive ex-U.S. commercialisation rights for systemic pegcetacoplan. Apellis has exclusive U.S. commercialisation rights for systemic pegcetacoplan. The companies have global co-development rights for systemic pegcetacoplan.

About Doptelet®
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 and the US Food & Drug Administration for the treatment of severe thrombocytopenia in adult patients with chronic liver disease who are scheduled to undergo an invasive procedure, and for the treatment of thrombocytopenia in adult patients with primary 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[1].

About Gamifant®
Gamifant® (emapalumab) is a monoclonal antibody that binds to and neutralises interferon gamma (IFNγ). In the US, emapalumab is indicated for the treatment of adult and paediatric (newborn 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). In September 2020, emapalumab received Orphan Drug Designation (ODD) by the FDA for prevention of graft failure following haematopoietic stem cell transplantation.

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 2020, Sobi's revenue amounted to SEK 15.3 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. You can find more information about Sobi at www.sobi.com.

Contacts
For details on how to contact the Sobi Investor Relations Team, please click here. For Sobi Media contacts, click here.

References
1. Lambert et al. Blood 2017.