PRESS RELEASE

Stockholm, Sweden, 10 June 2024

Sobi to present new data across its haematology portfolio at the 2024 EHA congress

Sobi® will present data at the EHA (European Haematology Association) hybrid congress, taking place in Madrid on the 13-16th June, highlighting the company's dedication to advancing treatments for rare and debilitating haematological diseases. The congress will feature Sobi's latest advances in the treatment of immune thrombocytopenia (ITP), myelofibrosis, haemophilia A, and paroxysmal nocturnal haemoglobinuria (PNH).

"We look forward to sharing new data related to several of Sobi's therapeutic areas at this year's EHA congress," said Lydia Abad-Franch, MD, Head of R&D and Medical Affairs, and Chief Medical Officer at Sobi. "Our ongoing efforts to enhance care and address the unique challenges faced by young patients with ITP will be highlighted with the primary data from the avatrombopag phase 3 paediatric study. Additional presentations will cover our advancements in treating a broad range of haematological conditions. We remain committed to raising the standard of care in rare haematological diseases and are excited to engage with our colleagues at this important event."

Key data to be presented at EHA 2024

Immune Thrombocytopenia		
Doptelet (avatrombopag)	A Phase 3, Randomised, Double-Blind, Placebo-controlled Trial to Evaluate the Efficacy and Safety of Avatrombopag for the Treatment of Children with Chronic Immune Thrombocytopenia (AVA-PED-301)	Oral presentation. #S318 Session: s416 Friday, June 14 14:45 - 16:00 CEST, Hall Mallo
	Phase 4 ADOPT Study: Interim Analysis of Efficacy and Safety Results of Avatrombopag Treatment in Adult Patients with Immune Thrombocytopenia	e-Poster. #P2235 Friday, June 14 09:00 CEST
Myelofibrosis		
VONJO (pacritinib)	A retrospective analysis of pacritinib treatment outcomes in myelofibrosis patients with and without monocytosis	e-Poster. #P2032 Friday, June 14 09:00 CEST
	Efficacy of pacritinib in patients with myelofibrosis who have overlapping thrombocytopenia and anemia	Poster session. #P1037 Friday, 14 June 18:00 - 19:00 CEST
	Real-World Treatment Patterns and Outcomes in Patients with Myelofibrosis Treated with Pacritinib in the United States	Poster session. #P1072 Friday, 14 June 18:00 - 19:00 CEST

Haemophilia A		
Efanesoctocog alfa	Individual Pharmacokinetic Evaluation of Fixed-Sequence Single-Dose Octocog Alfa, Rurioctocog Alfa Pegol, and Efanesoctocog Alfa in Adults With Severe Hemophilia A	Poster session. #P1645 Friday, June 14 18:00 - 19:00 CEST
Paroxysmal nocturnal haemoglobinuria		
Aspaveli (pegcetacoplan)	Efficacy and safety of intensive pegcetacoplan dosing for the treatment of acute hemolysis in patients with paroxysmal nocturnal hemoglobinuria	Poster session. #P812 Friday, June 14 18:00 - 19:00 CEST
	Long-term outcomes of pegcetacoplan treatment in patients with paroxysmal nocturnal hemoglobinuria and baseline hemoglobin levels greater than 10 grams per deciliter	Poster Session. #P816 Friday, June 14 18:00 - 19:00 CEST
	Characterization of clinically significant breakthrough hemolysis in patients with paroxysmal nocturnal hemoglobinuria treated with pegcetacoplan	Poster Session. #P819 Friday, June 14 18:00 - 19:00 CEST
	Thrombosis and Meningococcal infection rates in pegcetacoplan patients with Paroxysmal Nocturnal Hemoglobinuria in the Post-Marketing setting	Poster Session. #P838 Friday, June 14 18:00 - 19:00 CEST
	Improvement in Iron Overload with pegcetacoplan therapy in patients with Paroxysmal Nocturnal Hemoglobinuria previously treated with eculizumab	Poster Session. #P841 Friday, June 14 18:00 - 19:00 CEST

About Doptelet®
Doptelet (avatrombopag) 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 in the European Union, the United States and several other countries for the treatment of 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 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 and increased bleeding risk. 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 (Lambert et al. Blood 2017).

About VONJO®
VONJO is approved for the treatment of adults with intermediate- or high-risk primary or secondary (post-polycythemia vera or post-essential thrombocythemia) myelofibrosis with a platelet count below 50 × 109/L. This indication is approved under FDA accelerated approval based on spleen volume reduction. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s). CTI is conducting the Phase 3 PACIFICA study of VONJO in patients with myelofibrosis and severe thrombocytopenia as a post-marketing requirement. For more information, please visit https://www.ctibiopharma.com.

About efanesoctocog alfa
Efanesoctocog alfa [Antihemophilic Factor (Recombinant), Fc-VWF-XTEN Fusion Protein] (formerly BIVV001) is a new class of recombinant factor VIII therapy with the potential to deliver near-normal factor activity levels for a significant part of the week, improving 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 extend its time in circulation. It is the only therapy that has been shown to break through the von Willebrand factor ceiling, which imposes a half-life limitation on current factor VIII therapies. It is approved and marketed as ALTUVIIIO™ [Antihemophilic Factor (Recombinant), Fc-VWF-XTEN Fusion Protein-ehtl] by Sanofi in the United States, Japan, and Taiwan. The European Commission granted Orphan Drug designation in June 2019. The Committee for Medicinal Products for Human Use of the European Medicines Agency issued a positive opinion recommending approval of efanesoctocog alfa for the treatment and prevention of bleeds and perioperative prophylaxis in haemophilia A in April 2024.

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. It is approved for the treatment of paroxysmal nocturnal hemoglobinuria (PNH) in the United States, European Union, and other countries globally. The therapy is also under investigation for other rare diseases across haematology and nephrology.

About the Sobi and Apellis collaboration
Sobi and Apellis have global co-development rights for systemic pegcetacoplan. Sobi has exclusive ex-US commercialisation rights for systemic pegcetacoplan, and Apellis has exclusive US commercialisation rights for systemic pegcetacoplan and retains worldwide commercial rights for ophthalmological pegcetacoplan, including for geographic atrophy (GA).

About the Sanofi and Sobi collaboration
Sobi and Sanofi collaborate on the development and commercialisation of Alprolix® and Elocta®/Eloctate®. The companies also collaborate on the development and commercialisation of efanesoctocog alfa, or ALTUVIIIO™ in the US. 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.

About Sobi
Sobi® is a specialised international biopharmaceutical company transforming the lives of people with rare and debilitating diseases. Providing reliable access to innovative medicines in the areas of haematology, immunology and specialty care, Sobi has approximately 1,600 employees across Europe, North America, the Middle East, Asia and Australia. In 2022, revenue amounted to SEK 18.8 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. More about Sobi at sobi.com, LinkedIn and YouTube.

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