PRESS RELEASE

Stockholm, Sweden, 3 December 2024

Sobi®'s strength in haematology to be showcased at ASH 2024

New data from Sobi® and partners will be presented at the 66th Annual Meeting of the American Society of Hematology (ASH) in San Diego, CA (USA) from the 7th - 10th of December 2024. During the meeting several analyses will be presented on haemophilia A, haemophagocytic lymphohistiocytosis (HLH), myelofibrosis, paroxysmal nocturnal haemoglobinuria (PNH), and immune thrombocytopenia (ITP).

"Haemophilia A has a lasting impact on joint health and quality of life. At ASH 2024, three oral presentations will reveal important new data about long-term outcomes with ALTUVOCT® prophylaxis," said Lydia Abad-Franch, MD, MBA, Head of Research, Development, and Medical Affairs (RDMA), and Chief Medical Officer at Sobi. "We will also present new data spanning different severe and debilitating rare diseases, including an oral presentation on the real-world effectiveness of Doptelet® in treating people with ITP, demonstrating our broader commitment to advancing innovative treatments for people with rare haematological diseases. We look forward to sharing these findings in San Diego."

Key data to be presented at ASH 2024

Haemophilia A		
Altuvoct® (efanesoctocog alfa)	Association Between Hemophilia Joint Health Score and Quality of Life Using Results from the XTEND-1 Efanesoctocog Alfa Phase 3 Trial	Oral presentation. Session: 322 Session date: Monday, December 9, 2024, 10:30 AM - 12:00 PM Presentation time: 11:30 AM
	Clinical Outcomes Over 3 Years of Once-Weekly Efanesoctocog Alfa Treatment in Adults and Adolescents with Severe Hemophilia A: Second Interim Analysis from the Phase 3 XTEND-ed Long-Term Extension Study	Oral presentation. Session: 322. Session date: Monday, December 9, 2024, 10:30 AM - 12:00 PM Presentation time: 11:00 AM
	Real-world Experience of Switching to Prophylactic Efanesoctocog Alfa in Patients with Moderate or Severe Hemophilia A: An Analysis of the Adelphi Hemophilia Disease Specific Programme™	Oral presentation. Session: 322 Session date: Saturday, December 7, 2024, 12:00 PM - 1:30 PM Presentation Time: 1:00 PM
	Cost Comparison of Efanesoctocog Alfa with Existing Factor VIII Replacement Therapies for Major Surgeries in People with Severe Hemophilia A	Poster presentation. Session: 322 Session date: Sunday, December 8, 2024 Presentation time: 6:00 PM - 8:00 PM

General haemophilia A		
	Unmet Needs, Factor Consumption, and Healthcare Resource Use among People with Hemophilia A: Real-World Analysis of the Adelphi Hemophilia Disease Specific Programme™	Poster presentation. Session: 721 Session date: Sunday, December 8, 2024 Presentation time: 6:00 PM - 8:00 PM
	Real-World Patterns of Additnal Factor Treatment Use Among Hemophilia A Patients on Regular Prophylaxis in the United States: Results from the PicnicHealth database	Poster presentation. Session: 721 Session date: Sunday, December 8, 2024 Presentation time: 6:00 PM - 8:00 PM
Haemophagocytic Lymphohistiocytosis		
Gamifant® (emapalumab)	Transplant Related Outcomes in Patients with Hemophagocytic Lymphohistiocytosis Treated with Emapalumab as a Bridge to Hematopoietic Stem Cell Transplantation: The Real-HLH Study	Poster presentation. Session: 721 Session date: Sunday, December 8, 2024 Presentation time: 6:00 PM - 8:00 PM
	Patients (pts) with Optimized Hemophagocytic Lymphohistiocytosis (HLH) Inflammatory (OHI) Index-Confirmed Diagnosis of Malignancy-Associated HLH (mHLH) and Emapalumab Treatment	Poster presentation. Session: 201 Session date: Monday, December 9, 2024 Presentation time: 6:00 PM - 8:00 PM
Myelofibrosis		
Vonjo® (pacritinib)	Hematologic Improvement Experienced by Pacritinib-Treated Patients with Myelofibrosis in Real-World Clinical Settings	Poster presentation. Session: 908 Session date: Sunday, December 8, 2024 Presentation time: 6:00 PM - 8:00 PM
	Improvement in Serum Albumin as a Measure of Improved Metabolic Profile in Pacritinib-Treated Patients: a Retrospective Analysis of Patients Treated Across Three Clinical Trials	Poster presentation. Session: 634 Session date: Sunday, December 8, 2024 Presentation time: 6:00 PM - 8:00 PM
Paroxysmal Nocturnal Haemoglobinuria		
Aspaveli®/Empaveli ® (pegcetacoplan)	Real-World Clinical Outcomes for Complement inhibitor Experienced and Naïve Paroxysmal Nocturnal Hemoglobinuria Patients Prescribed Pegcetacoplan in Europe and Canada	Poster presentation. Session: 905 Session date: Monday, December 9, 2024 Presentation time: 6:00 PM - 8:00 PM

Immune Thrombocytopenia		
Doptelet® (avatrombopag)	Use of Avatrombopag in Patients with Immune Thrombocytopenia: Interim Analysis of the Phase 4 ADOPT Study	Oral presentation. Session: 311 Session date: Monday, December 9, 2024, 10:30 AM - 12:00 PM Presentation time: 11:45 AM
	Analysis of Durability of Response to Avatrombopag (AVA) from a Phase 3b Multicenter, Randomized, Double-Blind, Placebo (PBO)-controlled, Parallel-group Trial to Evaluate the Efficacy and Safety of AVA for the Treatment of Pediatric Patients with Immune Thrombocytopenia	Poster presentation. Session: 311 Session date: Saturday, December 7, 2024, Presentation time: 5:30 PM - 7:30 PM
	Enhanced Patient Satisfaction and Stability of Platelet Counts Following a Switch from Eltrombopag (ELT) or Romiplostim (ROMI) to Avatrombopag (AVA) In Adult Idiopathic Thrombocytopenic Purpura (ITP): Post-hoc Analyses from a Prospective Phase 4 Study	Poster presentation. Session: 311 Session date: Saturday, December 7, 2024, Presentation time: 5:30 PM - 7:30 PM
	Results of a Prospective, Open-label, Phase 4 Study Evaluating the Safety, Efficacy, and Treatment Satisfaction in Adult Immune Thrombocytopenia (ITP) Subjects after Switching to Avatrombopag (AVA) from Eltrombopag (ELT) or Romiplostim (ROMI)	Poster presentation. Session: 311 Session date: Saturday, December 7, 2024, Presentation time: 5:30 PM - 7:30 PM
	Real-World Treatment Patterns and Outcomes in Patients with Primary Immune Thrombocytopenia Treated with Avatrombopag in the United States: REAL-AVA 2.0 Interim	Poster presentation. Session: 905 Session date: Sunday, December 8, 2024 Presentation time: 6:00 PM - 8:00 PM

About ALTUVOCT®

ALTUVOCT® (efanesoctocog alfa) [Antihemophilic Factor (Recombinant), Fc-VWF-XTEN Fusion Protein] (formerly BIVV001) is the first high-sustained FVIII replacement 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. ALTUVOCT builds on the established Fc fusion technology by innovatively 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. The European Commission granted Orphan Drug designation in June 2019. It is approved and marketed as ALTUVOCT by Sobi in Europe. 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.

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 ALTUVOCT® (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 Gamifant®
Gamifant (emapalumab) is an anti-interferon gamma (IFNγ) monoclonal antibody that binds to and neutralises IFNγ. In the USA, Gamifant is indicated for the treatment of adult and paediatric (newborn and older) patients with primary haemophagocytic 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). Gamifant is indicated for administration through intravenous infusion over one hour twice per week until haematopoietic stem cell transplantation (HSCT).

About Vonjo®
Vonjo (pacritinib), a JAK-1 sparing JAK2/IRAK1/ACVR1 inhibitor, 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 × 10^9/L in the United States. 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). Currently, a Phase 3 study (PACIFICA) is being conducted to assess pacritinib in patients with myelofibrosis and severe thrombocytopenia as a post-marketing requirement.

About Aspaveli®/ Empaveli®
Aspaveli/Empaveli (pegcetacoplan) is a targeted C3 and C3b inhibitor 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. Pegcetacoplan is approved for the treatment of paroxysmal nocturnal haemoglobinuria (PNH) as Aspaveli/Empaveli in the United States, European Union, and other countries globally. Currently, a Phase 3 trial (VALIANT) is being conduted to assess the efficacy and safety of pegcetacoplan in patients with C3G and primary IC-MPGN. Aspaveli/Empaveli is also under investigation for several 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-U.S. commercialisation rights for systemic pegcetacoplan, and Apellis has exclusive U.S. commercialisation rights for systemic pegcetacoplan and worldwide commercial rights for ophthalmological pegcetacoplan, including for geographic atrophy.

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 over 30 countries worldwide, including the EU and the US, 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.

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 1,800 employees across Europe, North America, the Middle East, Asia, and Australia. In 2023, revenue amounted to SEK 22.1 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. More about Sobi at sobi.com and LinkedIn.

Contacts
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