# Press release

Stockholm, Sweden, 5 December 2025

## Sobi to Showcase Scientific advances and Commitment to Haematology at ASH 2025

Sobi® (STO: SOBI), today announced its participation at the 65th American Society of Hematology (ASH) Annual Meeting, taking place on 6 – 9 December in Orlando, Florida. At this year's meeting, Sobi will showcase its commitment to advancing care in haematology with 19 scientific abstracts, including two oral presentations. These feature the most recent clinical data and insights from completed and ongoing studies across Sobi's innovative portfolio including data from efanesoctocog alfa, pegcetacoplan, avatrombopag, emapalumab, and pacritinib. These presentations underscore Sobi's mission to deliver life-changing therapies for patients with rare and severe blood disorders.

"We will present evidence at ASH from a post hoc analysis demonstrating that pacritinib reduces or stabilises spleen size, improves haematologic parameters, and lessens myelofibrosis symptoms in patients with highrisk disease. These findings matter because they address key drivers of morbidity which affect daytoday functioning and quality of life. The ASH meeting also offers an opportunity to discuss the latest research on emapalumab, efanesoctocog alfa, pegcetacoplan, avatrombopag, as well as loncastuximab tesirine," said Lydia Abad-Franch, MD, Head of R&D and Medical Affairs, and Chief Medical Officer at Sobi.

**Summary of full Sobi data to be presented at ASH 2025:**

| **Efanesoctocog alfa** | |
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| Clinical outcomes up to four years of once-weekly Efanesoctocog Alfa Prophylaxis in previously treated adults, adolescents, and children with severe Haemophilia A: Interim analysis of the Phase 3 XTEND-ed long-term extension study | **Oral Presentation**<br>**Session Name:** 322. Haemophilia A and B: Clinical and epidemiological: Innovations shaping the future of Haemophilia care<br>**Date:** 7 December 2025<br>**Time:** 12:00 PM - 1:30 PM ET<br>**Presentation Time:** 1:00 PM - 1:15 PM ET<br>**Room:** OCCC – W304EFGH<br>**Publication Number:** 539 |
| Understanding unmet needs for people with Haemophilia A receiving factor and non-factor treatments | **Poster Presentation**<br>**Session Name:** Poster Session I<br>**Date:** 6 December 2025<br>**Time:** 5:30 PM - 7:30 PM ET<br>**Room:** OCCC - West Halls B3-B4 |

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| | **Publication Number:** 2679 |
| Real-world experience of Efanesoctocog Alfa in Haemophilia A patients in the US: A retrospective analysis | **Poster Presentation**<br>**Session Name:** Poster Session I<br>**Date:** 6 December 2025<br>**Time:** 5:30 PM - 7:30 PM ET<br>**Room:** OCCC - West Halls B3-B4<br>**Publication Number:** 1286 |
| Patient characteristics, treatment patterns, and bleeding in people with Haemophilia A without inhibitors initiating Efanesoctocog alfa in the US: An administrative claims analysis | **Poster Presentation**<br>**Session Name:** Poster Session I<br>**Date:** 6 December 2025<br>**Time:** 5:30 PM - 7:30 PM ET<br>**Room:** OCCC - West Halls B3-B4<br>**Publication Number:** 1290 |
| Quality of life and functional improvements with Efanesoctocog alfa in patients with moderate to severe Haemophilia A: A real-world survey | **Poster Presentation**<br>**Session name:** Poster Session III<br>**Date:** 8 December 2025<br>**Time:** 6:00 PM - 8:00 PM ET<br>**Room:** OCCC - West Halls B3-B4<br>**Publication Number:** 4846 |
| **Pegcetacoplan** | |
| Consistent benefits of Pegcetacoplan treatment in PNH patients with and without a history of Aplastic Anaemia in real world: Analysis of the ongoing COMPLETE Phase 4 observational study | **Poster Presentation**<br>**Session Name:** 101. Red Cells and Erythropoiesis, Excluding Iron: Poster I<br>**Date:** 6 December 2025<br>**Time:** 5:30 PM - 7:30 PM ET<br>**Room:** OCCC - West Halls B3-B4 |
| Early results from the ongoing Pegcetacoplan Silo of the International Paroxysmal Nocturnal Haemoglobinuria Interest Group Registry | **Poster Presentation**<br>**Session Name:** 508. Bone Marrow Failure: Acquired: Poster II<br>**Date:** 7 December 2025<br>**Time:** 6:00:00 PM – 6:00:00 PM ET<br>**Location:** OCCC - West Halls B3-B4 |
| Real-world clinical characteristics and treatment outcomes in PNH patients prescribed Pegcetacoplan: Insights of complement inhibitor-experienced and -naïve patients across Europe, the United States and Canada | **Poster Presentation**<br>**Session Name:** 508. Bone Marrow Failure: Acquired: Poster III<br>**Date:** 8 December 2025<br>**Time:** 6:00 PM - 8:00 PM ET<br>**Room:** OCCC - West Halls B3-B4 |
| **Pegcetacoplan - Publication Only Abstracts** | |
| Real-world effectiveness of Pegcetacoplan in Paroxysmal Nocturnal Haemoglobinuria: A systematic review of clinical and patient-reported outcomes | Publication only - published online on 3 November 2025, at 9:00 AM ET |
| Low risk for Meningococcal and encapsulated bacteria infections with systemically administered | |

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| Pegcetacoplan in Paroxysmal Nocturnal Haemoglobinuria and C3 Glomerulopathies | |
| Overview of treatment advances with complement Inhibitors in patients with Paroxysmal Nocturnal Haemoglobinuria | |
| Optimising PNH treatment with the complement inhibitor Pegcetacoplan: A case report | |
| User experience with Pegcetacoplan on-body Injector in patients with Paroxysmal Nocturnal Hemoglobinuria | |
| **Avatrombopag** | |
| Real-world treatment patterns and outcomes among patients with immune thrombocytopenia (ITP) who switched treatment from Eltrombopag or Romiplostim to Avatrombopag in the United States: Results from the real-AVA 3.5 study | **Poster Presentation**<br>**Session Name:** 905. Outcomes research: Non-malignant conditions excluding Hemoglobinopathies: Poster I<br>**Date:** 6 December 2025<br>**Time:** 5:30 PM - 7:30 PM ET<br>**Room:** OCCC - West Halls B3-B4 |
| Real-world safety and efficacy of Avatrombopag in adults with Immune Thrombocytopenia: A systematic review and meta-analysis. | **Global Abstract**<br>**Session Name:** 311. Disorders of platelet number or function: Clinical and epidemiological: Poster I<br>**Date:** 6 December 2025<br>**Time:** 5:30 PM – 7:30 PM ET<br>**Room:** OCCC – West Halls B3-B4 |
| Patient-reported outcomes of Avatrombopag for Chronic Immune Thrombocytopenia: Interim analysis of the Phase 4 ADOPT Study | **Poster Presentation**<br>**Session Name:** 905. Outcomes research: Non-malignant conditions excluding Hemoglobinopathies: Poster III<br>**Date:** 8 December 2025<br>**Time:** 6:00 PM - 8:00 PM ET<br>**Room:** OCCC - West Halls B3-B4 |
| **Emapalumab** | |
| Use of Emapalumab is associated with rapid and sustained benefits in pHLH subgroups, including CNS involvement and previously untreated patients: Pooled analysis of prospective trials NI-0501-04, NI-050105 and NI-050109 | **Poster Presentation**<br>**Session Name:** 201. Granulocytes, Monocytes, and Macrophages: Poster II<br>**Date:** 7 December 2025<br>**Time:** 6:00 PM - 8:00 PM ET<br>**Room:** OCCC - West Halls B3-B4 |
| Emapalumab induces rapid, durable responses and reliable bridging to curative HSCT in patients with primary HLH: Pooled analysis of prospective trials NI-0501-04, NI-0501-05 and NI-0501-09 | **Poster Presentation**<br>**Session Name:** 201. Granulocytes, Monocytes, and Macrophages: Poster III<br>**Date:** 8 December 2025<br>**Time:** 6:00 PM - 8:00 PM ET<br>**Room:** OCCC - West Halls B3-B4 |
| **Pacritinib** | |

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| Real-world treatment patterns and outcomes in patients with myelofibrosis who presented with thrombocytopenia and anaemia at initiation of Pacritinib treatment | **Oral Presentation**<br>**Session Name:** 908. Outcomes Research: Myeloid Malignancies: Real-World Experiences<br>**Session date:** 7 December 2025<br>**Session time:** 4:30 PM - 6:00 PM ET<br>**Presentation time:** 5:30 PM - 5:45 PM<br>**Room:** OCCC - W414CD |
| Pacritinib in patients with high-risk myelofibrosis: Outcomes from post-hoc analyses of two Phase 3 studies | **Poster Presentation**<br>**Session Name:** 634. Myeloproliferative Syndromes: Clinical and epidemiological: Poster I<br>**Date:** 6 December 2025<br>**Time:** 5:30 PM - 7:30 PM ET<br>**Room:** OCCC - West Halls B3-B4 |
| Real-world treatment patterns and clinical outcomes in patients with Myelofibrosis treated with Pacritinib (PAC): Results from the MY-PAC Study | **Poster Presentation**<br>**Session Name:** 908. Outcomes research: Myeloid Malignancies: Poster II<br>**Date:** 7 December 2025<br>**Time:** 6:00 PM - 8:00 PM ET<br>**Room:** OCCC - West Halls B3-B4 |
| PROSPERA (ABNL-MARRO 002): A randomised Phase 2 study of Pacritinib vs. Hydroxyurea in patients with Advanced Proliferative Chronic Myelomonocytic Leukaemia (CMML) | **Poster Presentation**<br>**Session Name:** 637. Myelodysplastic Syndromes: Clinical and epidemiological: Poster II<br>**Date:** 7 December 2025<br>**Time:** 6:00 PM - 8:00 PM ET<br>**Room:** OCCC - West Halls B3-B4 |
| Treatment patterns and outcomes in patients with myelofibrosis treated with Pacritinib following a switch from Ruxolitinib: The MY-PAC Study | **Session Name:** 634. Myeloproliferative Syndromes: Clinical and epidemiological: Poster III<br>**Date:** 8 December 2025<br>**Time:** 6:00 PM - 8:00 PM ET<br>**Room:** OCCC - West Halls B3-B4 |
| Incidence, prevalence, and clinical outcomes of Myelofibrosis with and without Cytopenia in the United States | |

**About ALTUVOCT ® (efanesoctocog alfa)**
ALTUVOCT® (efanesoctocog alfa) is indicated for the treatment and prophylaxis of bleeding in patients with haemophilia A (HA). ALTUVOCT can be used for all age groups and any disease severity.

**About the Sobi and Sanofi Collaboration**
Sobi and Sanofi 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.

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**About Aspaveli®/Empaveli® (pegcetacoplan)**
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. Aspaveli/Empaveli is approved for the treatment of adults with paroxysmal nocturnal haemoglobinuria (PNH) in the US, European Union, and other countries globally, and for C3 glomerulopathy (C3G) and primary immune complex membranoproliferative glomerulonephritis (IC-MPGN) in the United States. It is under regulatory review for C3G and primary IC-MPGN in the European Union and other countries globally.

**About the Sobi and Apellis Collaboration**
Apellis and Sobi have global co-development rights for systemic pegcetacoplan. Sobi has exclusive ex-U.S. commercialisation rights for systemic pegcetacoplan, and its opt-in rights for future development programs are unchanged, exercisable at any time prior to commercialisation. Apellis has exclusive U.S. commercialisation rights for systemic pegcetacoplan and worldwide commercial rights for ophthalmological pegcetacoplan, including for geographic atrophy.

**About Doptelet® (avatrombopag)**
Doptelet (avatrombopag) is indicated for the treatment of primary chronic immune thrombocytopenia (ITP) in adult patients who are refractory to other treatments, and for the treatment of severe thrombocytopenia in adult patients with chronic liver disease (CLD) scheduled to undergo an invasive procedure. Doptelet is also approved for the treatment of chronic ITP in pediatric patients.

**About Gamifant® (emapalumab-lzsg)**
Gamifant is an anti-IFNγ antibody that binds free and receptor-bound IFNγ, which when secreted in an uncontrolled manner can cause hyperinflammation. Gamifant is indicated for intravenous infusion over one hour and is FDA approved for the treatment of primary hemophagocytic lymphohistiocytosis (HLH) and macrophage activation syndrome (MAS) in Still's disease.

**About Vonjo® (pacritinib)**
Vonjo (pacritinib) is a kinase inhibitor indicated in the United States 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. This indication is approved under accelerated approval based on spleen volume reduction. Continued approval may be contingent upon verification and description of clinical benefit in confirmatory trials. Vonjo is also being investigated for other rare hematologic conditions, including VEXAS syndrome.

**Sobi®**
Sobi is a global biopharma company unlocking the potential of breakthrough innovations, transforming everyday life for people living with rare diseases. Sobi has approximately 1,900 employees across Europe, North America, the Middle East, Asia and Australia. In 2024, revenue amounted to SEK 26 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. More about Sobi at sobi.com and LinkedIn.

**Contacts**
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