PRESS RELEASE

Stockholm, Sweden, June 1 2021

Sobi at EHA virtual congress 2021: focus on results in PNH and treatment for ITP

Swedish Orphan Biovitrum AB (publ) (Sobi™) will present data at the EHA (European Haematology Association) virtual congress, 9-17 June 2021, highlighting the company's advancement of treatments for rare haematological diseases. During the thematic days of the congress Sobi will host two events: an update in haematology on chronic immune thrombocytopenia (ITP) and an expert roundtable on paroxysmal nocturnal haemoglobinuria (PNH). Both events are open for all registered participants at the EHA congress, an event that brings haematologists together to consolidate knowledge and experience gained worldwide.

"We are committed to transforming the lives of people living with rare diseases; the breadth of our activity and our presentations at EHA are important contributions to expanding the understanding and knowledge of the community in support of our commitment", says Ravi Rao, Head of R&D and Chief Medical Officer at Sobi.

PEGASUS 48-week data on pegcetacoplan presented for the first time

Results from the PEGASUS 48-week clinical study of pegcetacoplan for PNH will be presented for the first time. PEGASUS evaluated the efficacy and safety of pegcetacoplan in adult patients with suboptimal response to prior treatment with eculizumab. Data from PEGASUS will be presented orally, as a poster and in published abstracts.

Oral presentation on topline PEGASUS 48-week data

- Forty-eight-week efficacy and safety of pegcetacoplan in adult patients with paroxysmal nocturnal hemoglobinuria and suboptimal response to prior eculizumab treatment (S174). Friday, 11 June (9:00 CEST/ 3:00 AM EST)

Expert roundtable

- PNH Expert Roundtable, Tuesday, 15 June (9.45-10.30 CEST)

e-Poster presentation

- Effect of pegcetacoplan on quality of life in patients with paroxysmal nocturnal hemoglobinuria: Week 48 of PEGASUS phase 3 trial comparing pegcetacoplan to eculizumab (EP595)
- Paroxysmal nocturnal hemoglobinuria´s humanistic and economic burden in patients receiving C5 inhibitors in Europe (EP1191)

Published abstracts

- Categorized hematologic response to pegcetacoplan versus eculizumab in patients with paroxysmal nocturnal hemoglobinuria: Post hoc analysis of PEGASUS phase 3 randomized trial data (PB1477)
- Comparative effectiveness of pegcetacoplan versus ravulizumab in patients with paroxysmal nocturnal hemoglobinuria: A matching-adjusted indirect comparison using 26-week PEGASUS phase 3 trial data (PB1485)
- Injection-site reactions at week 48 in the randomized phase 3 PEGASUS trial of pegcetacoplan compared with eculizumab for individuals with paroxysmal nocturnal hemoglobinuria (PB1475)
- Long-term effects in subgroups of patients with paroxysmal nocturnal hemoglobinuria treated with pegcetacoplan versus eculizumab: 48-week analysis of PEGASUS phase 3 trial (PB1471)

Real-world data for Doptelet® in the treatment of chronic immune thrombocytopenia

Sobi will present abstracts on the use of Doptelet (avatrombopag) in chronic immune thrombocytopenia (ITP), including real-world data from the United States.

Update in Haematology

- ITP Update in Haematology, Sunday 13 June (18.00-19.30 CEST)

e-Poster presentation

- A multicenter U.S. study of avatrombopag switch therapy following prior eltrombopag or romiplostim. Abstract Code: EP1144
- Durability of platelet count response in patients treated with avatrombopag for immune thrombocytopenia (ITP): Post-hoc results from the phase 3 core and open-label extension study. Abstract Code: EP1148
- Length of thrombopoietin receptor agonist (TPO-RA) treatment and persistence in immune thrombocytopenia (ITP): Real world United States claims analyses. Abstract Code: EP1151

About pegcetacoplan

Pegcetacoplan is a therapy targeting C3, the central protein in the complement cascade. It acts proximally in the complement cascade controlling both C3bmediated extravascular haemolysis and terminal complementmediated intravascular haemolysis. Pegcetacoplan is being evaluated in several clinical studies across haematology, ophthalmology, nephrology, and neurology. In May 2021, pegcetacoplan was approved as EMPAVELI™ in the US for the treatment of adults with paroxysmal nocturnal haemoglobinuria (PNH). The marketing authorisation application for pegcetacoplan for paroxysmal nocturnal haemoglobinuria (PNH) is under review by the European Medicines Agency (EMA). Pegcetacoplan was also granted Fast Track designation by the FDA for the treatment of geographic atrophy and received orphan drug designation for the treatment of C3 glomerulopathy by the FDA and EMA. For additional information regarding pegcetacoplan clinical studies, visit apellis.com/our-science/clinical-trials.

About Doptelet® (avatrombopag)
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 EMA and FDA for the treatment of thrombocytopenia in adult patients with chronic liver disease who are scheduled to undergo a 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. 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 the Sobi and Apellis Collaboration
Sobi and Apellis entered a collaboration to develop and commercialise systemic pegcetacoplan in October 2020. The companies have global co-development rights for systemic pegcetacoplan. Sobi has exclusive ex-US commercialisation rights for systemic pegcetacoplan, while Apellis has exclusive US commercialisation rights for systemic pegcetacoplan and retains worldwide commercial rights for ophthalmological pegcetacoplan, including for geographic atrophy (GA).

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, Middle East and Asia. In 2020, Sobi's revenues amounted to SEK 15.3 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. You can find more information about Sobi at sobi.com.

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[1] (Lambert et al. Blood 201)