PRESS RELEASE

Stockholm, Sweden, 13 December 2021

Aspaveli®/Empaveli™ (pegcetacoplan) demonstrated sustained normalisation of clinical measures in a broad PNH patient population

- *Improvements demonstrated in treatment-naïve patients and patients with baseline haemoglobin levels greater than or equal to 10.0 g/dL*
- *Data were presented at the American Society of Hematology (ASH) Annual Meeting*

Swedish Orphan Biovitrum AB (publ) (Sobi™) (STO:SOBI) and Apellis Pharmaceuticals, Inc. (Nasdaq: APLS) announced today new data demonstrating that Aspaveli®/Empaveli™ (pegcetacoplan) provides consistent, sustained normalisation of clinical measures across patients with paroxysmal nocturnal haemoglobinuria (PNH) who are treatment-naïve and patients with baseline haemoglobin levels greater than or equal to 10.0 g/dL. The data were presented at the American Society of Hematology Annual Meeting (ASH) taking place 11 – 14 December 2021.

Sustained normalisation and superior improvements of clinical measures in treatment-naïve patients
New data from the PRINCE phase 3 study in treatment-naïve patients with PNH showed that treatment with pegcetacoplan resulted in sustained and superior improvements in the co-primary endpoints of haemoglobin stabilisation through week 26 and reduction in lactate dehydrogenase (LDH) compared to standard of care, which did not include complement inhibitors, at week 26.

Improvements were seen as early as two weeks after starting treatment with pegcetacoplan and patients showed sustained normalisation across key markers of disease through week 26:

- 46 per cent of pegcetacoplan patients achieved haemoglobin normalisation in the absence of transfusions vs. 0 per cent for standard of care ($p<0.0010$), reaching a mean haemoglobin level of 12.8 g/dL from a mean baseline of 9.4 g/dL
- Mean LDH levels rapidly fell from 9.5x the upper limit of normal (ULN) to below 1.5x the ULN by week two, normalised by week four, and were maintained through week 26 with pegcetacoplan
- 91 per cent of pegcetacoplan patients achieved transfusion avoidance vs. 6 per cent for standard of care ($p<0.0001$), demonstrating superiority

The safety profile of pegcetacoplan was consistent with previous studies. At week 26, 9 per cent of patients in the pegcetacoplan group experienced a serious adverse event (SAE) compared to 17 per cent on standard of care. No cases of thrombosis or meningococcal infection were reported in either group. The most common adverse events reported during the study in the pegcetacoplan and standard of care groups, respectively, were injection site reaction (30% vs. 0%), hypokalemia (13% vs. 11%), dizziness (11% vs. 0%) and fever (9% vs. 0%).

"The data presented at ASH add to a robust body of evidence that underscores the consistent efficacy and safety of pegcetacoplan across a broad range of adults with PNH," said Federico Grossi, M.D., Ph.D., Chief Medical Officer of Apellis. "Pegcetacoplan has the potential to elevate the standard of care for adults with PNH regardless of prior treatment or baseline haemoglobin levels."

Clinically meaningful improvements in patients with near-normal baseline haemoglobin levels
A new post hoc analysis across studies from the pegcetacoplan PNH clinical development program showed that pegcetacoplan-treated patients with baseline haemoglobin levels greater than or equal to 10.0 g/dL demonstrated clinically meaningful improvements across key markers of disease. The analysis included data from patients who were treatment-naïve and patients that remained anaemic despite stable treatment with eculizumab, a C5 inhibitor.

Detailed data showed pegcetacoplan:

- increased mean haemoglobin levels to 13.9 g/dL, 12.1 g/dL, and 12.7 g/dL from a mean baseline of 11.3 g/dL, 10.2 g/dL, and 10.4 g/dL in the PRINCE, PEGASUS, and PADDOCK studies, respectively
- demonstrated mean improvements from baseline in the Functional Assessment of Chronic Illness Therapy (FACIT)-fatigue score of 9.9 points, 13.3 points, and 6.6 points in the PRINCE, PEGASUS, and PADDOCK studies. A three-point improvement is generally considered to be clinically meaningful

"The presented data reinforce the efficacy and safety profile of pegcetacoplan in PNH," said Ravi Rao, Head of Research & Development and Chief Medical Officer at Sobi. "We are committed to improve care, and to make a difference in the lives of people with this rare blood disease."

About the PRINCE study
The PRINCE study (NCT04085601) was a 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 by 2 g/dL or more from their baseline value.

About the PEGASUS study
The PEGASUS study (NCT03500549) was a multi-centre, randomised, head-to-head phase 3 study in 80 adults with paroxysmal nocturnal haemoglobinuria (PNH). The primary objective of this study was to establish the efficacy and safety of pegcetacoplan compared to eculizumab. Participants must have been on eculizumab (stable for at least three months) with a haemoglobin level of <10.5 g/dL at the screening visit. During the four-week run-in, patients were dosed with 1080 mg of pegcetacoplan twice weekly (n=41) in addition to their current dose of eculizumab. During the 16-week randomised, controlled period, patients were randomised to receive either 1080 mg of pegcetacoplan twice weekly or their current dose of eculizumab (n=39). All participants completing the randomised controlled period (n=77) opted to enter the open-label pegcetacoplan treatment period.

About the PADDOCK study
PADDOCK (NCT02588833) was a multi-centre, open-label, multiple ascending dose, phase 1b study in 23 adults with paroxysmal nocturnal haemoglobinuria (PNH) who have never received eculizumab. The primary objective of this study, designed with two cohorts, was to establish the safety and efficacy of 270 mg of pegcetacoplan administered daily by subcutaneous injection in adults with PNH. Patients in cohort one received a suboptimal dose of 180 mg of pegcetacoplan once daily for 28 days and subjects in cohort two received 270 mg of pegcetacoplan once daily for up to one year.

About Aspaveli®/Empaveli™ (pegcetacoplan)
Empaveli™/Aspaveli® (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. Pegcetacoplan 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, which is the European trade name for pegcetacoplan, 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 Paroxysmal Nocturnal Haemoglobinuria (PNH)
PNH is a rare, chronic, life-threatening blood disorder characterised by the destruction of oxygen-carrying red blood cells through extravascular and intravascular haemolysis. Persistently low haemoglobin can result in frequent transfusions and debilitating symptoms such as severe fatigue, haemoglobinuria and difficulty breathing (dyspnoea).

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 Apellis
Apellis Pharmaceuticals, Inc. is a global biopharmaceutical company that is committed to leveraging courageous science, creativity, and compassion to deliver life-changing therapies. Leaders in targeted C3 therapies, Apellis aim to develop transformative therapies for a broad range of debilitating diseases that are driven by excessive activation of the complement cascade, including those within hematology, ophthalmology, nephrology, and neurology. For more information, please visit http://apellis.com.

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. More about Sobi at sobi.com, LinkedIn and YouTube.

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