PRESS RELEASE

Stockholm, Sweden, 10 December 2020

Sobi and Apellis report positive top-line results at 48 weeks from the phase 3 PEGASUS study of pegcetacoplan in PNH

- Treatment with pegcetacoplan resulted in a sustained improvement in haemoglobin with a mean increase from baseline of 2.7 g/dL at week 48, which is equal to the 2.7 g/dL increase seen at week 16 with pegcetacoplan-treated patients
- Sustained improvements in transfusion avoidance, reticulocyte count, lactate dehydrogenase (LDH) level and Functional Assessment of Chronic Illness Therapy (FACIT)-fatigue score were observed in patients treated with pegcetacoplan
- Safety profile of pegcetacoplan was consistent with previously reported data

Stockholm, Sweden and Waltham MA, USA - Swedish Orphan Biovitrum AB (publ) (Sobi™) (STO:SOBI) and Apellis Pharmaceuticals Inc. (Nasdaq: APLS) today announced positive top-line results at week 48 from the phase 3 PEGASUS study, which demonstrated sustained haematological and clinical improvements in patients with paroxysmal nocturnal haemoglobinuria (PNH) who were treated with pegcetacoplan, an investigational, targeted C3 therapy. The safety profile of pegcetacoplan was consistent with previously reported data and no new safety signals were identified.

All patients (n=77) who completed the 16-week randomized controlled period of the PEGASUS study, which evaluated pegcetacoplan compared to eculizumab, entered the open-label period and received pegcetacoplan from week 17 to week 48.

At week 48, haemoglobin increases were sustained in pegcetacoplan-treated patients with a mean improvement from baseline of 2.7 g/dL which is equal to the 2.7 g/dL mean increase seen at week 16 with pegcetacoplan-treated patients.

Additionally, eculizumab-treated patients who switched to pegcetacoplan during the open-label period experienced sustained improvements in haemoglobin and other haematological and clinical measures, similar to patients treated with pegcetacoplan monotherapy during the randomized controlled period.

“These long-term results show that pegcetacoplan has the potential to help patients with PNH gain and maintain more complete control of the disease,” said Federico Grossi, M.D., Ph.D., chief medical officer of Apellis. “The sustained haematologic and quality-of-life improvements and consistent safety profile of pegcetacoplan observed in this study adds to a growing body of evidence that demonstrates the potential of this investigational, targeted C3 therapy to elevate the standard of care and improve the lives of people with PNH.”

In addition to a sustained improvement in haemoglobin, patients treated with pegcetacoplan maintained improvements across key secondary endpoints. Throughout the 48-week study, 73 per cent of patients treated with pegcetacoplan remained transfusion free. For comparison, 25 per cent of patients were transfusion free over the year prior to entering the PEGASUS study while on treatment with eculizumab. Improvements across additional markers of disease, such as reticulocyte count, lactate dehydrogenase (LDH) levels and the Functional Assessment of Chronic Illness Therapy (FACIT)-fatigue scores, were maintained.

Overall, the safety profile of pegcetacoplan was consistent with previously reported data throughout the 48-week study. 24 of 80 pegcetacoplan monotherapy-treated patients (30 per cent) experienced a serious adverse event (SAE); five of the SAEs (6 per cent) were assessed to be possibly related to study treatment. No cases of meningitis were reported. One death was reported due to COVID-19 and was unrelated to study treatment. The most common adverse events (AEs) reported throughout the study were injection site reactions (36 per cent), haemolysis (24 per cent), and diarrhoea (21 per cent). 12 out of 80 patients (15 per cent) discontinued due to adverse events, with five discontinuations due to haemolysis. 64 of the 67 patients (96 per cent) who completed the open-label period opted to enter the extension study.

“Despite existing treatments, many patients with PNH continue to suffer from persistently low haemoglobin, which can lead to a need for frequent transfusions and debilitating fatigue,” said Ravi Rao, Head of R&D and Chief Medical Officer at Sobi. “The long-term data suggest that pegcetacoplan, if approved has the potential to provide meaningful and durable benefits to these patients with high unmet medical need.”

Marketing applications for pegcetacoplan for the treatment of PNH are under review by the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA). The FDA granted the application Priority Review designation and set a target action date of May

14, 2021. An opinion from the Committee for Medicinal Products for Human Use (CHMP) is expected in 2021.

Detailed data will be presented at a future medical congress.

About the PEGASUS Study

The PEGASUS study (APL2-302; NCT03500549) is a multi-center, randomized, 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 randomized, controlled period, patients were randomized to receive either 1080 mg of pegcetacoplan twice weekly or their current dose of eculizumab (n=39). All participants completing the randomized controlled period (n=77) opted to enter the open-label pegcetacoplan treatment period.

About pegcetacoplan

Pegcetacoplan is an investigational, 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 a synthetic cyclic peptide conjugated to a polyethylene glycol polymer that binds specifically to C3 and C3b. Pegcetacoplan is being evaluated in several clinical studies across haematology, ophthalmology, nephrology, and neurology. Marketing applications for pegcetacoplan for paroxysmal nocturnal haemoglobinuria (PNH) are under review by the U.S. Food and Drug Administration (FDA), which has granted the application Priority Review designation, and the European Medicines Agency (EMA). Pegcetacoplan was 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 Paroxysmal Nocturnal Haemoglobinuria (PNH)

PNH is a rare, chronic, life-threatening blood disorder characterized 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 (dyspnea). A retrospective analysis shows that, even on eculizumab, approximately 72 per cent of people with PNH have anemia, a key indicator of ongoing haemolysis.[1] The analysis also finds that 36 per cent of patients require one or more transfusions a year and 16 per cent require three or more.[1]

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, we 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 haematology, ophthalmology, nephrology, and neurology. For more information, please visit www.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, the Middle East, Russia and North Africa. In 2019, Sobi's revenues amounted to SEK 14.2 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. You can find more information about Sobi at www.sobi.com.

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