PRESS RELEASE

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Positive top-line results from the phase 3 PRINCE study of pegcetacoplan in treatment-naïve patients with PNH

- *Pegcetacoplan demonstrated statistical superiority on the co-primary endpoints of haemoglobin stabilisation (p<0.0001) and reduction in lactate dehydrogenase (LDH) (p<0.0001) compared to standard of care, which did not include complement inhibitors, at week 26*
- *Mean haemoglobin levels in the pegcetacoplan group increased from 9.4 g/dL to 12.1 g/dL compared to an increase from 8.7 g/dL to 9.4 g/dL on standard of care (p=0.0019)*
- *91 per cent of patients on pegcetacoplan were transfusion free compared to 22 per cent on standard of care (p<0.0001)*
- *The safety profile of pegcetacoplan was consistent with previous studies*

Swedish Orphan Biovitrum AB (publ) (Sobi™) and Apellis Pharmaceuticals, Inc. (Nasdaq: APLS) (GLOBE NEWSWIRE) today reported positive top-line results from the phase 3 PRINCE study evaluating the efficacy and safety of pegcetacoplan in adults with paroxysmal nocturnal haemoglobinuria (PNH) who are treatment naïve, meaning they had not received a complement inhibitor within three months before entering the study.

Pegcetacoplan demonstrated statistical superiority on the co-primary endpoints of haemoglobin stabilization and reduction in lactate dehydrogenase (LDH) compared to standard of care, which did not include complement inhibitors, at week 26.

- 86 per cent of pegcetacoplan treated patients achieved haemoglobin stabilisation compared to 0 per cent of patients on standard of care (p<0.0001). Haemoglobin stabilisation was defined as an avoidance of a >1 g/dL decrease in haemoglobin levels in the absence of transfusions.
- Mean LDH in the pegcetacoplan group decreased by 90 per cent from a baseline of 2151 U/L [9.5x upper limit of normal (ULN)] to 211 U/L, which is within the normal range, compared to a 14 per cent reduction on standard of care from a baseline of 1946 U/L (8.6x ULN) to 1681 U/L (7.4x ULN) (p<0.0001).

“The positive PRINCE data showed that pegcetacoplan provided clinically meaningful improvements across multiple measures that are important for patients and build on our recent FDA approval of pegcetacoplan in PNH,” said Federico Grossi, M.D., Ph.D., chief medical officer, Apellis. “Combined with previous studies, these results emphasize the potential of pegcetacoplan to provide disease control for all adults with PNH regardless of prior treatment.”

Pegcetacoplan also achieved statistical superiority on several secondary endpoints, including improvements in haemoglobin levels and transfusion avoidance, compared to standard of care, which did not include complement inhibitors.

- Mean haemoglobin levels in the pegcetacoplan group increased from 9.4 g/dL to 12.1 g/dL compared to an increase from a baseline of 8.7 g/dL to 9.4 g/dL on standard of care (p=0.0019).
- 91 per cent of patients on pegcetacoplan were transfusion free compared to 22 per cent on standard of care (p<0.0001).

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. One death was reported in each group, and neither were related to treatment. No cases of meningitis or thrombosis 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 per cent vs. 0 per cent), hypokalemia (13 per cent vs. 11 per cent), and fever (9 per cent vs. 0 per cent).

“The PRINCE study results reinforce the efficacy and safety profile of pegcetacoplan in PNH,” said Ravi Rao, Head of Research & Development and Chief Medical Officer at Sobi. “Our hope is to contribute to an improvement of care, and to make a difference in the lives of people with this rare blood disease.”

Detailed results from the PRINCE study will be presented at medical congresses.

About the PRINCE study

The PRINCE study (NCT04085601) is a 2:1 (pegcetacoplan: standard of care) randomized, multi-center, 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 randomized, 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 2 g/dL or more from their baseline value.

About pegcetacoplan

Pegcetacoplan is an investigational 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 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).

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 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. You can find more information about Sobi at sobi.com.

This information is information that Swedish Orphan Biovitrum AB (publ) is obliged to make public pursuant to the EU Market Abuse Regulation. The information was submitted for publication, through the agency of the contact persons set out below, at 13:00 CEST on 25 May 2021.

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