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The New England Journal of Medicine publishes phase 3 PEGASUS study results comparing pegcetacoplan to eculizumab for PNH

- Pegcetacoplan, an investigational targeted C3 therapy for serious, complement-driven diseases, demonstrated superiority to eculizumab with a statistically significant improvement in haemoglobin levels and showed improvements in key clinical outcomes
- Marketing applications for pegcetacoplan are under Priority Review with the FDA and under review with the EMA

STOCKHOLM, Sweden and WALTHAM, Mass, USA - Swedish Orphan Biovitrum AB (publ) (Sobi™) (STO:SOBI) and Apellis Pharmaceuticals, Inc. (Nasdaq: APLS) today announced that The New England Journal of Medicine (NEJM) published results from the phase 3 PEGASUS study, which demonstrated the superiority of pegcetacoplan, an investigational targeted C3 therapy, in improving haemoglobin levels and showed improvements in key clinical outcomes compared to eculizumab, a C5 inhibitor, in adults with paroxysmal nocturnal haemoglobinuria (PNH) at 16 weeks who had persistent anaemia following treatment with eculizumab.

“The data published in The New England Journal of Medicine underscore the potential of pegcetacoplan to be a significant advancement for people living with PNH,” said Peter Hillmen, M.B., Ch.B., Ph.D, professor of experimental haematology at Leeds Teaching Hospitals NHS Trust and PEGASUS study author. “There is still a need for new treatments because many patients with PNH treated with C5 inhibitors today remain anemic, resulting in moderate to severe fatigue, with a proportion continuing to require transfusions.”

“The PEGASUS study results demonstrate that, if approved, pegcetacoplan has the potential to elevate the standard of care for PNH by providing more complete disease control,” said Federico Grossi, M.D., Ph.D., Chief Medical Officer of Apellis. “We are working to quickly bring this potential new treatment to PNH patients and to advance development of pegcetacoplan for many other serious, complement-driven diseases.”

“The NEJM publication of the PEGASUS study results reflect the significance of these data for both the medical and PNH patient communities,” said Ravi Rao, Head of Research & Development and Chief Medical Officer at Sobi. “The results further advance our understanding
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of the role of C3 in PNH and our hope is to contribute to the improvement and care for PNH patients around the world”.

The results published in NEJM highlight that pegcetacoplan met the study’s primary endpoint for efficacy, demonstrating superiority to eculizumab with a statistically significant improvement in adjusted means of 3.8 g/dL of haemoglobin at week 16 (p<0.001). Additionally, 85% of pegcetacoplan-treated patients were transfusion free over 16 weeks versus 15% of eculizumab-treated patients. There were meaningful improvements across key markers of disease such as absolute reticulocyte count, lactate dehydrogenase (LDH), and fatigue as measured by the Functional Assessment of Chronic Illness Therapy (FACIT)-fatigue score.

Safety was comparable between pegcetacoplan and eculizumab in this study. Seven of 41 patients (17%) in the pegcetacoplan group experienced a SAE, and 6 of 39 patients (15%) in the eculizumab group experienced SAEs. No cases of meningitis and no deaths were reported in either treatment group.

The most common adverse events reported during the 16-week, randomized, controlled treatment period in the pegcetacoplan and eculizumab groups, respectively, were injection site reactions (37% vs. 3%), diarrhoea (22% vs. 3%), breakthrough haemolysis (10% vs. 23%), headache (7% vs. 23%), and fatigue (5% vs. 15%).

Top-line results from the PEGASUS study at 48 weeks were recently announced, which showed sustained improvements in key markers of disease as well as a consistent safety profile with previously reported data.

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.

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
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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 U.S. Food and Drug Administration (FDA) for the treatment of geographic atrophy and received orphan drug designation for the treatment of C3G by the FDA and European Medicines Agency. For additional information regarding pegcetacoplan clinical trials, visit https://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% of people with PNH have anemia, a key indicator of ongoing haemolysis.\(^1\) The analysis also finds that 36% of patients require one or more transfusions a year and 16% 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 http://apellis.com.

About Sobi
Sobi is a specialized 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, Asia and North Africa. In 2020, Sobi’s revenue amounted to SEK 15.3 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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