PRESS RELEASE

Stockholm, Sweden, 10 June 2022

New data reinforce robust efficacy and safety profile of Aspaveli/Empaveli (pegcetacoplan) for PNH at the EHA2022 Congress

Swedish Orphan Biovitrum AB (publ) (Sobi®) (STO:SOBI) and Apellis Pharmaceuticals, Inc. (Nasdaq: APLS) today reported new analyses of phase 3 studies that reinforce the robust efficacy and safety profile of Aspaveli®/Empaveli® (pegcetacoplan) for paroxysmal nocturnal haemoglobinuria (PNH). The data will be presented at the hybrid European Hematology Association (EHA) Congress in Vienna, Austria.

New analyses demonstrated that treatment with Aspaveli/Empaveli resulted in meaningful improvements in quality of life for treatment-naïve patients and suggested the incidence of thrombosis was comparable to eculizumab, a C5 inhibitor. Additionally, a matching-adjusted indirect comparison (MAIC) showed significant improvements in clinical outcomes in treatment-naïve patients who received Aspaveli/Empaveli compared to C5 inhibitors.

“We are very pleased that these new data further reinforce the safety and efficacy of Aspaveli/Empaveli in treating such a rare, chronic and life-threatening condition,” said Anders Ullman, Head of Research & Development and Chief Medical Officer at Sobi. “Sobi and Apellis are firmly committed to improving the care and quality of life for those affected by this rare blood disease.”

“The data presented at EHA add to a growing body of evidence, which shows that Empaveli/Aspaveli leads to both clinically meaningful efficacy and improved quality of life regardless of prior treatment,” said Peter Hillmen, M.B. Ch.B., Ph.D., Head of haematology engagement at Apellis. “Many patients experience a significant disease burden, even with C5 inhibitor treatment, so these data further emphasise that Empaveli/Aspaveli has the potential to become a new standard of care for PNH.”

Aspaveli/Empaveli demonstrated meaningful improvements in quality of life in treatment-naïve patients

In an analysis of the PRINCE phase 3 study, Aspaveli/Empaveli patients who were previously treatment-naïve demonstrated meaningful quality-of-life improvements through 26 weeks, reaching normal or near-normal levels of the general population. These data, which were assessed using multiple measures including the European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire-Core 30 scale, will be reported during an oral presentation at the congress.

Results suggested incidence of thrombosis comparable across Aspaveli/Empaveli- and eculizumab-treated patient groups

A post-hoc analysis of data from all PNH clinical trials of Aspaveli/Empaveli revealed that there were 1.54 thrombotic events per 100 patient-years for Aspaveli/Empaveli-treated patients compared to 1.77 thrombotic events per 100 patient-years for eculizumab-treated patients prior to entry in the PEGASUS phase 3 study.

Additionally, D-dimer normalisation was comparable across Aspaveli/Empaveli- and eculizumab-treated patient groups in a post-hoc analysis of the phase 3 studies. D-dimer is a marker of thrombotic risk, one of the most common life-threatening complications of PNH.

Aspaveli/Empaveli demonstrated significant improvements in clinical outcomes versus C5 inhibitors in treatment-naïve patients

Using a MAIC methodology, individual patient data from the PRINCE phase 3 study were compared to aggregate, published data from the ALXN1210-PNH-301 study[1], which compared C5 inhibitors ravulizumab and eculizumab in PNH patients who were treatment-naïve.

Patients treated with Aspaveli/Empaveli showed significant improvements compared to C5 inhibitors across all key disease measures evaluated, including lactate dehydrogenase normalisation, haemoglobin stabilisation and transfusion avoidance at week 26.

In the absence of a clinical head-to-head study, MAIC is a valid and accepted method for comparative effectiveness research used by health technology assessment bodies across the world.[2, 3] As with other MAIC analyses, matching may not adjust for all confounding factors due to differences inherent in study design and entry criteria. Key limitations include differences in the route of administration, treatment administration schedule and dosing regimen.

About Aspaveli®/ Empaveli®
Aspaveli/Empaveli (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. It is approved as Aspaveli® for the treatment of adults with paroxysmal nocturnal haemoglobinuria (PNH) who are anaemic after treatment with a C5 inhibitor for at least three months in the European Union and the United Kingdom, and as Empaveli® for treatment of adult patients with PNH in the United States and Saudi Arabia. Empaveli is also approved in Australia for treatment of adult patients with PNH who have an inadequate response to, or are intolerant of, a C5 inhibitor.

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 Aspaveli/Empaveli in patients who had 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 Aspaveli/Empaveli 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 Aspaveli/Empaveli 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, open-label, 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 Aspaveli/Empaveli 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 Aspaveli/Empaveli 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 Aspaveli/Empaveli 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 Aspaveli/Empaveli treatment period.

About the matching-adjusted indirect comparison analysis
Using a matching-adjusted indirect comparison (MAIC) methodology, individual patient data from the PRINCE study were compared to aggregate, published results from the ALXN1210-PNH-301 study,[3] which compared the C5 inhibitors ravulizumab and eculizumab among patients with PNH who were naïve to complement-inhibitor treatment. To adjust for cross-study differences in baseline characteristics, propensity score weighting was used to balance demographic and clinical characteristics. Outcomes assessed from the PRINCE study at week 26 and the ALXN1210-PNH-301 study at week 26 included lactate dehydrogenase (LDH) levels, LDH normalisation, haemoglobin stabilisation, and transfusion avoidance. As with other MAIC analyses, matching may not adjust for all confounding factors due to differences inherent in study design and entry criteria.

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 complement, they ushered in the first new class of complement medicine in 15 years with the approval of the first and only targeted C3 therapy. They are advancing this science to continually develop transformative medicines for people living with rare, retinal, and neurological diseases. For more information, please visit http://apellis.com or follow us on Twitter and LinkedIn.

References
1. Lee JW, Sicre de Fontbrune F, Wong Lee Lee L, et al. Ravulizumab (ALXN1210) vs eculizumab in adult patients with PNH naive to complement inhibitors: the 301 study. Blood. 2019;133(6):530-539. doi:10.1182/blood-2018-09-876136.
2. Phillippo DM, Ades AE, Dias S, Palmer S, Abrams KR, and Welton NJ. Methods for Population-Adjusted Indirect Comparisons in Health Technology Appraisal. Medical Decision Making 2018;38(2): 200-211.
3. Signorovitch JE, Sikirica V, Erder MH, Xie J, Lu M, Hodgkins PS, Betts KA, and Wu EQ. Matching-Adjusted Indirect Comparisons: A New Tool for Timely Comparative Effectiveness Research. Value in Health 2012;15(6):940-947.

Sobi®
Sobi is a specialised international biopharmaceutical company transforming the lives of people with rare diseases. Providing sustainable access to innovative medicines in the areas of haematology, immunology, and specialty care, Sobi has approximately 1,600 employees across Europe, North America, the Middle East, and Asia. In 2021, revenue amounted to SEK 15.5 billion. Sobi's share (STO: SOBI) is listed on Nasdaq Stockholm. More about Sobi at sobi.com, LinkedIn and YouTube.

Contacts Sobi
For details on how to contact the Sobi Investor Relations Team, please click here. For Sobi Media contacts, click here.

Contacts Apellis
To contact the Apellis Investor Relations or Media contacts, please click here.