

PRESS RELEASE

Stockholm, Sweden, 19 November 2020

Sobi and Apellis announce first patient dosed in potentially registrational ALS study of pegcetacoplan

- 52-week phase 2 MERIDIAN study to evaluate pegcetacoplan, a targeted C3 therapy, in approximately 200 adults with ALS

Stockholm, Sweden and Waltham MA, USA - Swedish Orphan Biovitrum AB (publ) (Sobi™) (STO:SOBI) and Apellis Pharmaceuticals, Inc. (Nasdaq: APLS) today announced that the first patient has been dosed in the potentially registrational phase 2 MERIDIAN study of pegcetacoplan, a targeted C3 therapy, in approximately 200 adults with sporadic amyotrophic lateral sclerosis (ALS).

Studies suggest that elevated levels of C3 may play a role in the progression of ALS, a neurodegenerative disease that leads to progressive muscle weakness and paralysis. The MERIDIAN study will assess whether pegcetacoplan may offer a new treatment approach for people living with ALS by controlling complement activation at the level of C3. There are currently no treatments to slow the advance of ALS.

“ALS patients have a very high unmet need. They expect more and better treatment options,” said Bashar Al-Nakhala, Chief Operations Officer of the ALS Therapeutic Development Institute. “We are pleased that Apellis and Sobi have joined the ALS clinical development community with our shared goal of halting the devastating progression of ALS.”

“We are delighted that the first patient in the phase 2 clinical study has been dosed as there is an urgency for a treatment for patients with ALS” said Ravi Rao, Head of R&D and Chief Medical Officer at Sobi. “In collaboration with Apellis, we look forward to evaluating the potential of pegcetacoplan in patients with ALS.”

“Based on the suspected role of C3 in ALS, we are working urgently to understand whether pegcetacoplan, a targeted C3 therapy, has the potential to slow disease progression and make a difference for the ALS community,” said Federico Grossi, M.D., Ph.D, Chief Medical Officer of Apellis. “We designed the MERIDIAN study based on significant feedback from the community, and we are dedicated to continuing our partnership to one day bring a meaningful therapy to families living with ALS.”

The phase 2 MERIDIAN study (APL2-ALS-206) is a potentially registrational, randomized, double-blind, placebo-controlled, multicenter study designed to evaluate the efficacy and safety of pegcetacoplan in approximately 200 adults with sporadic ALS. Study participants will be randomized in a 2:1 ratio to receive pegcetacoplan or placebo while continuing to receive their existing standard of care treatment for ALS. After 52 weeks of blinded treatment, all patients in the study will receive pegcetacoplan. To reduce the burden on people living with ALS and their caregivers, the study has

been designed to minimize the number of in-clinic visits, with approximately six clinic visits in the first year and four in the open-label second year.

The primary endpoint of the study is the Combined Assessment of Function and Survival (CAFS) rank scores at week 52. Key secondary endpoints include measures of lung function, muscle strength, and quality of life. For more information about the phase 2 MERIDIAN study, visit www.clinicaltrials.gov (NCT04579666).

About amyotrophic lateral sclerosis (ALS)

ALS is a devastating neurodegenerative disease that results in progressive muscle weakness and paralysis due to the death of nerve cells, called motor neurons, in the brain and spinal cord.^{1,2} The death of motor neurons leads to the progressive loss of voluntary muscle movement required for speaking, walking, swallowing, and breathing.^{1,2} In individuals with ALS, high levels of C3 are present at the neuromuscular junction³ where motor neurons communicate directly to muscle cells. Numerous studies suggest that elevated levels of C3 present throughout the motor system of ALS patients are likely to contribute to chronic neuroinflammation and the death of motor neurons.^{3,4,5} There are currently no approved treatments that stop or reverse the progression of ALS, which impacts ~225,000 patients worldwide.⁶

About pegcetacoplan (APL-2)

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 also granted Fast Track designation by the FDA for the treatment of PNH and 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 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 www.sobi.com.

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¹ National Institute of Neurological Disorders and Stroke. (2020). Amyotrophic Lateral Sclerosis Fact Sheet. Retrieved from <https://www.ninds.nih.gov/Disorders/Patient-Caregiver-Education/Fact-Sheets/Amyotrophic-lateral-Sclerosis-ALS-Fact-Sheet>

² ALS Association. What is ALS? Retrieved June 2020 from <https://www.als.org/understanding-als/what-is-als>

³ Bahia El Idrissi N, et al. J Neuroinflammation. 2016;13(1):72-4 Sta M, et al. Neurobiol Dis. 2011;42(3):211-220.

⁴ Woodruff, et al., PNAS January 7, 2014 111 (1) E3-E4

⁵ Lee, et al Journal of Neuroinflammation volume 15: 171 (2018)25 Arthur K et al. Nat Commun, 2016, Vol 7, article 12408

⁶ Arthur K et al. Nat Commun, 2016, Vol 7, article 12408