PRESS RELEASE

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First patient dosed in the VALIANT phase 3 study of pegcetacoplan for IC-MPGN and C3G, rare kidney diseases with high unmet medical need

Sobi® (STO:SOBI) and Apellis Pharmaceuticals, Inc. (Nasdaq: APLS) today announced that the first patient has been dosed in the VALIANT phase 3 study investigating pegcetacoplan, a targeted C3 therapy, in primary immune-complex membranoproliferative glomerulonephritis (IC-MPGN) and C3 glomerulopathy (C3G), two rare and debilitating kidney diseases with similar underlying causes and no approved treatment.

“Individuals with IC-MPGN and C3G experience a high burden of disease, due to the lack of approved treatments to slow or stop disease progression. These diseases often lead to kidney failure, requiring dialysis or a kidney transplant, imposing further substantial burdens,” said Kristen Hood, MSN, RN, Executive Director of Research Engagement at Nephcure Kidney International. “We are excited that Apellis and Sobi are advancing a phase 3 study of pegcetacoplan in patients aged 12 and older, with primary IC-MPGN or C3G, including those with post-transplant recurrence.”

Uncontrolled activation of the complement cascade, a part of the body’s immune system, is believed to play a critical role in the progression of IC-MPGN and C3G where excessive accumulation of C3 breakdown products in the kidney causes inflammation and organ damage.[1-4] It is estimated that up to 8,000 people in Europe and 5,000 in the United States are living with IC-MPGN or C3G[5], and approximately 50 per cent ultimately suffer from kidney failure within five to ten years of diagnosis.[6]

“Sobi and Apellis are the only companies actively pursuing a treatment for IC-MPGN," said Anders Ullman, MD, PhD, Head of Research and Development and Chief Medical Officer at Sobi. “We are pleased that the first patient has been dosed in this phase 3 study for IC-MPGN and C3G, both of which represent an extremely high unmet medical need. We are committed to bringing a meaningful treatment to patients affected by these severe diseases.”

“The positive phase 2 results supported the advancement of pegcetacoplan to a confirmatory phase 3 study in IC-MPGN and C3G,” said Jeffrey Eisele, PhD, Chief Development Officer at Apellis. “There is a significant need for treatments for people living with these rare kidney diseases, and we believe pegcetacoplan, by targeting complement centrally at C3, may have the potential to protect kidney function and preserve patient quality of life.”

About the VALIANT study

The VALIANT phase 3 study (NCT05067127) is a randomised, placebo-controlled, double-blinded, multi-centre study designed to evaluate pegcetacoplan efficacy and safety in approximately 90 patients who are 12 years of age and older with primary IC-MPGN or C3G. It is the only study to include both native kidney patients and patients who have recurrent disease after receiving a kidney transplant. Study participants will be randomised to receive 1080 mg of pegcetacoplan or placebo twice weekly for 26 weeks. Following this 26-week randomised, controlled period, patients will proceed to a 26-week open-label phase in which all patients receive pegcetacoplan. The primary endpoint of the study is the proportion of study participants with a reduction from baseline in urine protein-to-creatinine ratio (uPCR) of at least 50 per cent at week 26. uPCR is an important indicator of kidney function.

About immune-complex membranoproliferative glomerulonephritis (IC-MPGN) and C3 glomerulopathy (C3G)
IC-MPGN and C3G are rare, debilitating kidney diseases that are estimated to affect up to 8,000 people in Europe and 5,000 in the United States.[5] There are no approved therapies for the diseases, and symptoms include blood in the urine, dark foamy urine due to the presence of protein, swelling, and high blood pressure.[7] Approximately 50 per cent of people living with IC-MPGN and C3G ultimately suffer from kidney failure within five to ten years of diagnosis.[6] There are no treatments available that target the underlying complement-mediated mechanism of these diseases and prevent loss of kidney function, before or after renal transplant. Although IC-MPGN is considered a distinct disease from C3G, the underlying cause and progression of the two diseases are remarkably similar and include overactivation of the complement cascade, with excessive accumulation of C3 breakdown products in the kidney causing inflammation and damage to the organ.[1-4,8]

About pegcetacoplan in rare diseases
Pegcetacoplan is a targeted C3 therapy designed to regulate excessive activation of the complement cascade, a part of the body's immune system, which can lead to the onset and progression of many serious diseases. Pegcetacoplan is under investigation for several rare diseases across haematology, nephrology, and neurology. Pegcetacoplan 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 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, we ushered in the first new class of complement medicine in 15 years with the approval of the first and only targeted C3 therapy. We 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.

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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.

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