PRESS RELEASE

Stockholm, Sweden, 8 August 2024

Sobi and Apellis announce positive topline results from phase 3 VALIANT study of pegcetacoplan in C3G and primary IC-MPGN

- *Met the primary endpoint, achieving statistically significant 68% (p<0.0001) reduction in proteinuria compared to placebo in a broad study population*
- *Positive results consistent across all subgroups, including C3G and IC-MPGN, adolescent and adult patients, and native and post-transplant kidneys*
- *Demonstrated favourable safety, consistent with established profile*
- *Companies plan to submit data for regulatory approval in the US and EU*

Sobi® (STO: SOBI) and Apellis Pharmaceuticals, Inc. (Nasdaq: APLS) today announced positive topline results from the Phase 3 VALIANT study investigating systemic pegcetacoplan in patients with C3 glomerulopathy (C3G) or primary immune complex membranoproliferative glomerulonephritis (IC-MPGN), which are rare kidney diseases with no approved treatments.

The study met the primary endpoint, demonstrating a statistically significant and clinically meaningful 68% (p<0.0001) proteinuria reduction (log-transformed ratio of urine protein-to-creatinine ratio) in C3G and IC-MPGN patients treated with pegcetacoplan compared to placebo, both in addition to background therapy, at Week 26. Results were consistent across all subgroups including C3G and IC-MPGN, adolescent and adult patients, and native and post-transplant kidneys.

Pegcetacoplan also demonstrated statistical significance on the key secondary endpoints of composite renal endpoint, which combines proteinuria reduction and estimated glomerular filtration rate (eGFR) stabilisation, and proteinuria reduction of at least 50% compared to baseline, as well as nominal significance on the histological endpoint of reduction in C3c staining on kidney biopsy and stabilisation of kidney function as measured by eGFR compared to placebo.

“As a clinician, I’m thrilled by these groundbreaking results, which show that pegcetacoplan has the potential to significantly improve the lives of patients with C3G and IC-MPGN, regardless of disease type, age, and transplant status,” said Carla Nester, M.D. MSA, FASN, lead principal investigator for the VALIANT study and Jean E. Robillard M.D., professor of paediatric nephrology, University of Iowa Stead Family Children's Hospital. “Currently, many patients living with these rare diseases will eventually require a kidney transplant or lifelong dialysis, so there is an urgent need for a treatment that targets the underlying cause of these diseases. These positive data are a major advance for the rare kidney disease community.”

"Today’s announcement further strengthens our belief in pegcetacoplan’s potential to meet the critical needs of patients with these severe and life-threatening kidney conditions," stated Lydia Abad-Franch, MD, Head of R&D, Medical Affairs, and Chief Medical Officer at Sobi. "We remain committed to progressing pegcetacoplan's development and expanding its reach, driven by our steadfast mission to transform the lives of those affected by rare diseases."

“These results exceeded our already high expectations. Pegcetacoplan is the first investigational therapy to show such a strong reduction in proteinuria in C3G and IC-MPGN with supportive data

across multiple measures of disease activity," said Jeffrey Eisele, Ph.D., Chief Development Officer at Apellis. "Building on pegcetacoplan's approval in PNH, we look forward to sharing these data with the FDA and working quickly to bring this treatment to patients with these debilitating kidney diseases."

In the VALIANT study, pegcetacoplan demonstrated favourable safety and tolerability, consistent with its established profile. Rates of adverse events (AEs), serious AEs, and AEs leading to study drug discontinuation were similar between the pegcetacoplan and placebo groups. There were no cases of meningitis or serious infections attributed to encapsulated bacteria.

All patients who have already completed the VALIANT study have now enrolled into the VALE long-term extension study.

Sobi plans to submit a marketing application with the European Medicines Agency (EMA) in 2025. Apellis also plans to submit a supplemental new drug application to the U.S. Food and Drug Administration (FDA) in early 2025. Detailed data will be presented at an upcoming medical congress.

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 124 patients who are 12 years of age and older with C3G or primary IC-MPGN. It is the largest single trial conducted in these populations and the only study to include adolescent and adult patients, with native and post-transplant kidneys. Study participants were randomised to receive 1080 mg of pegcetacoplan or placebo twice weekly for 26 weeks. Following this 26-week randomised controlled period, patients were able to proceed to a 26-week open-label phase in which all patients receive pegcetacoplan. The primary endpoint of the study was the log transformed ratio of urine protein-to-creatinine ratio (uPCR) at Week 26 compared to baseline.

About C3 Glomerulopathy (C3G) and primary Immune-Complex Membranoproliferative Glomerulonephritis (IC-MPGN)

C3G and primary IC-MPGN are rare and debilitating kidney diseases that can lead to kidney failure. Excessive C3c deposits are a marker of disease activity, which can lead to kidney inflammation, damage, and failure. There are no treatments that target the underlying cause of these diseases. Approximately 50% of people living with C3G and primary IC-MPGN suffer from kidney failure within five to 10 years of diagnosis, requiring a burdensome kidney transplant or lifelong dialysis.[1] Additionally, two-thirds of patients who previously received a kidney transplant will experience disease recurrence.[2] The diseases are estimated to affect 5,000 people in the United States and up to 8,000 in Europe.[3]

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 rare diseases across haematology and nephrology. Pegcetacoplan is approved for the treatment of paroxysmal nocturnal haemoglobinuria (PNH) as EMPAVELI®/Aspaveli® in the United States, European Union, and other countries globally.

About the Apellis and Sobi Collaboration

Apellis and Sobi have global co-development rights for systemic pegcetacoplan. Sobi has exclusive ex-U.S. commercialisation rights for systemic pegcetacoplan, and Apellis has exclusive U.S. commercialisation rights for systemic pegcetacoplan and worldwide commercial rights for ophthalmological pegcetacoplan, including for geographic atrophy.

About Apellis

Apellis Pharmaceuticals, Inc. is a global biopharmaceutical company that combines courageous science and compassion to develop life-changing therapies for some of the most challenging diseases patients face. We ushered in the first new class of complement medicine in 15 years and now have two approved medicines targeting C3. These include the first-ever therapy for geographic atrophy, a leading cause of blindness around the world. We believe we have only begun to unlock the potential of targeting C3 across serious retinal, rare, and neurological diseases. For more information, please visit http://apellis.com or follow us on X (Twitter) and LinkedIn.

About Sobi®
Sobi® is a specialised international biopharmaceutical company transforming the lives of people with rare and debilitating diseases. Providing reliable access to innovative medicines in the areas of haematology, immunology, and specialty care, Sobi has approximately 1,800 employees across Europe, North America, the Middle East, Asia, and Australia. In 2023, revenue amounted to SEK 22.1 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. More about Sobi at sobi.com and LinkedIn.

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

References
1. C3 glomerulopathy. National Institute of Health, Genetics Home Reference. https://ghr.nlm.nih.gov/condition/c3-glomerulopathy#resources. Accessed November 21, 2019.
2. Zand L, et al Clinical findings, pathology, and outcomes of C3GN after kidney transplantation. J Am Soc Nephrol. 2014 May;25(5):1110-7. doi: 10.1681/ASN.2013070715. Epub 2013 Dec 19.
3. Data on file using literature consensus.

This information is information that Sobi is obliged to make public pursuant to the EU Market Abuse Regulation. The information was submitted for publication, through the agency of the contact person set out below, on 8 August 2024 at 13:00 CEST.

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