PRESS RELEASE

Stockholm, Sweden, 26 October 2024

Pivotal VALIANT results presented at Kidney Week show pegcetacoplan treatment effect in patients with C3G / primary IC-MPGN

- *Statistically significant 68% (p<0.0001) proteinuria reduction compared to placebo, with reduction observed as early as Week 4*

- *All secondary endpoints favoured treatment with pegcetacoplan, including:*
  - *Stabilised eGFR, a key measure of kidney function*
  - *71% of patients achieved zero C3c staining intensity, indicating clearance of C3c deposits*

- *Results consistent across subgroups including C3G and primary IC-MPGN, adolescent and adult patients, and native and post-transplant kidneys*

Sobi® (STO: SOBI) and Apellis Pharmaceuticals, Inc. today announced that detailed data from the phase 3 VALIANT study were presented as an oral presentation during the High-Impact Clinical Trials session at the 2024 American Society of Nephrology (ASN) Kidney Week. The results highlighted the potential of systemic pegcetacoplan treatment in patients with C3 glomerulopathy (C3G) and primary immune complex membranoproliferative glomerulonephritis (IC-MPGN), which are rare, debilitating kidney diseases.

“Pegcetacoplan is the only treatment to achieve substantial and clinically meaningful effects across all key markers of disease: proteinuria, eGFR stabilisation, and C3c staining,” said Carla Nester, M.D. MSA, FASN, lead principal investigator for the VALIANT study, professor of internal medicine and paediatrics, and director of paediatric nephrology, University of Iowa Stead Family Children's Hospital. “C3G and primary IC-MPGN often affect patients as early as adolescence, often leading to either a kidney transplant or lifelong dialysis, so there is an urgent need for an approved treatment that can prolong kidney function.”

Statistically significant 68% proteinuria reduction across a broad study population, with reduction observed as early as Week 4

Pegcetacoplan-treated patients showed a statistically significant and clinically meaningful 68.1% (p<0.0001) proteinuria reduction (log-transformed ratio of urine protein-to-creatinine ratio) compared to placebo, both in addition to standard of care therapy, at Week 26. The proteinuria reduction was observed as early as Week 4 and continued through the six-month treatment period. Proteinuria reduction was consistent across broad patient subgroups including adolescent and adult patients, C3G and primary IC-MPGN patients, and patients with native and post-transplant kidneys.

Pegcetacoplan stabilised eGFR and demonstrated substantial reduction in C3c staining

Patients treated with pegcetacoplan achieved stabilisation of estimated glomerular filtration rate (eGFR), a key measure of kidney function, with a difference of +6.3 mL/min/1.73 m^2 (95% CI 0.5, 12.1; nominal p value =0.03) over six months compared to placebo.

Additionally, a substantial proportion of patients treated with pegcetacoplan also demonstrated a reduction in C3c staining intensity. Excessive C3c deposits are a key marker of disease activity which can lead to kidney inflammation, damage, and failure.

- 74.3% of patients in the pegcetacoplan group and 11.8% on placebo achieved a reduction in C3c staining intensity by two or more orders of magnitude from baseline, resulting in 27-fold higher odds ratio of achieving this reduction with pegcetacoplan (nominal p value <0.0001).
- 71.4% of pegcetacoplan-treated patients achieved zero C3c staining intensity, indicating clearance of C3c deposits.

"C3G and primary IC-MPGN can have a severe impact on patients' lives, frequently resulting in kidney failure. The findings presented at ASN further highlight the potential of pegcetacoplan to preserve kidney function and address these unmet medical needs. We extend our heartfelt gratitude to the participants, their families, and the dedicated healthcare professionals who made this study possible," stated Lydia Abad-Franch, MD, Head of R&D, Medical Affairs, and Chief Medical Officer at Sobi.

All secondary endpoints favoured treatment with pegcetacoplan

In addition to the positive results on proteinuria, eGFR and C3c staining, pegcetacoplan demonstrated statistical significance on the key secondary endpoints of composite renal endpoint, which combines proteinuria reduction and eGFR stabilisation, and proteinuria reduction of at least 50% compared to baseline, as well as a numerical improvement in the C3G histologic index activity score.

During the randomised, controlled 26-week treatment period, pegcetacoplan demonstrated favourable safety and tolerability, as well as a high compliance rate, consistent with its established profile. Rates of treatment-emergent adverse events (AEs) (84.1% in pegcetacoplan vs. 93.4% in placebo), serious AEs (9.5% vs. 9.8%), severe AEs (4.8 % vs. 6.6%), and AEs leading to study discontinuation (1.6% vs. 1.6%) were similar between the pegcetacoplan and placebo groups. There were no cases of meningococcal meningitis or serious infections attributed to encapsulated bacteria. Patients who complete the VALIANT study are eligible to enrol into the VALE long-term extension study.

Sobi plans to submit a marketing application with the European Medicines Agency in 2025. Apellis plans to submit a supplemental new drug application to the U.S. Food and Drug Administration in early 2025.

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 pegcetacoplan or placebo twice weekly for 26 weeks. Following this 26-week randomised controlled period, patients are 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 key 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 IC-MPGN suffer from kidney failure within five to 10 years of diagnosis, requiring a burdensome kidney transplant or lifelong dialysis.[1] Additionally, 90% 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 Sobi and Apellis collaboration
Sobi and Apellis 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 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.

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