PRESS RELEASE

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Positive data from phase 2 NOBLE study of pegcetacoplan presented as late-breaker at ERA congress

- *Rapid reduction in disease activity seen at 12 weeks sustained at one year*
- *55% of patients showed zero C3c staining intensity, indicating that C3c deposits cleared*
- *Highlighted as late-breaking oral presentation at the European Renal Association (ERA) Congress*

Sobi® and Apellis Pharmaceuticals, Inc. today announced positive one-year results from the phase 2 NOBLE study investigating systemic pegcetacoplan, a targeted C3 therapy, for the treatment of post-transplant recurrence of C3 glomerulopathy (C3G) and primary immune complex membranoproliferative glomerulonephritis (IC-MPGN). These data were presented during a late-breaking oral presentation at the European Renal Association (ERA) Congress taking place May 23-26 in Stockholm, Sweden.

“It is exciting to see that treatment with pegcetacoplan rapidly reduced disease activity in only 12 weeks and sustained the effects over the long term,” said Fadi Fakhouri, M.D., PhD, presenting author and professor of nephrology at CHUV Lausanne, Switzerland. “Post-transplant C3G and IC-MPGN patients are likely to experience disease recurrence, putting them at risk of needing another kidney transplant or dialysis. There is a huge need for a treatment that targets the cause of these diseases, and I am very encouraged by these results.”

At one year, of the 11 pegcetacoplan-treated patients with available data:

- Seven (64%) patients showed a reduction in C3c staining by two or more orders of magnitude of intensity from baseline.
- Six (55%) patients, including the three IC-MPGN patients, showed zero C3c staining intensity, indicating that C3c deposits were cleared.
- Consistent with C3c staining reduction, seven patients (64%) showed zero inflammation as measured by the activity score of the C3G histologic index.

Excessive C3c deposits are a marker of disease activity, which can lead to kidney inflammation, damage, and failure. Clearance of both C3c deposits and inflammation allow the kidney to recover and prolong the function of the kidney. Additionally, treatment with pegcetacoplan resulted in sustained improvements in key measures of disease including proteinuria and continued stabilisation of kidney function.

"Patients with post-transplant C3G and IC-MPGN often face disease relapse, so the need for a therapeutic approach addressing the root cause of these conditions cannot be overstated,” said Lydia Abad-Franch MD, Head of R&D and Medical Affairs, and Chief Medical Officer at Sobi. “These findings underscore our conviction in pegcetacoplan’s potential to address these rare, severe, and life-threatening conditions in both native and post-transplant kidneys."

"The NOBLE data further indicate that pegcetacoplan is treating the underlying cause of these diseases by directly targeting C3," said Caroline Baumal, M.D., Chief Medical Officer, Apellis. "Our ongoing Phase 3 VALIANT study evaluates the potential of pegcetacoplan in all patients with these rare kidney diseases, and we look forward to sharing the topline results later this year."

Pegcetacoplan was generally well-tolerated. The majority of adverse events were mild to moderate and consistent with previously reported results.

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.

About the Phase 2 NOBLE Study

The Phase 2 NOBLE study (NCT04572854) is a multicentre, open-label, randomised, controlled study designed to evaluate the efficacy and safety of pegcetacoplan in 13 adults who have post-transplant recurrence of C3G or IC-MPGN. Study participants were randomised in a 3:1 ratio to receive pegcetacoplan (IC-MPGN: n=2; C3G: n=8) or maintain standard of care (IC-MPGN: n=1; C3G: n=2) for 12 weeks, and all patients received pegcetacoplan from week 13 to week 52. The primary endpoint of the study was the proportion of patients with reduction in C3c staining on renal biopsy after 12 weeks of treatment with pegcetacoplan. Secondary endpoints include an evaluation of safety, the proportion of patients with reduction in C3c staining on renal biopsy after 52 weeks of treatment, and the proportion of patients achieving at least a 50% reduction in proteinuria.

About the VALIANT Study

The VALIANT Phase 3 study (NCT05067127) is a randomised, placebo-controlled, double-blinded, multicentre study designed to evaluate pegcetacoplan efficacy and safety in approximately 90 patients who are 12 years of age and older with C3G or primary IC-MPGN. It is the only study to include both patients with native kidney disease 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 log transformed ratio of urine protein-to-creatinine ratio (uPCR) at week 26 compared to baseline.

About C3 Glomerulopathy (C3G) and 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 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. commercialization 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. Learn more about Sobi at sobi.com and LinkedIn.

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