PRESS RELEASE

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EMA validates indication extension application for Aspaveli® for treatment of C3G and primary IC-MPGN

Sobi® and Apellis Pharmaceuticals today announced the European Medicines Agency (EMA) has validated an indication extension application for Aspaveli® (pegcetacoplan) for the treatment of C3 glomerulopathy (C3G) and primary immune complex membranoproliferative glomerulonephritis (IC-MPGN), which are rare, chronic kidney diseases with no approved treatments.

"C3G and IC-MPGN are severe and life-threatening kidney conditions, often leading to kidney failure and requiring a kidney transplant or dialysis for life," said Lydia Abad-Franch MD, Head of R&D and Medical Affairs, and Chief Medical Officer at Sobi. "With no approved treatments currently available, this important milestone reflects our dedication to improving outcomes for those affected by these rare kidney conditions."

The submission includes positive data from the Phase 3 VALIANT study. The study met its primary endpoint demonstrating a statistically significant and clinically meaningful 68% (p<0.0001) proteinuria reduction in pegcetacoplan-treated patients compared to placebo. At week 26, results were consistent across all subgroups, including disease type, age, and transplant status. Additionally, pegcetacoplan-treated patients achieved stabilisation of kidney function (nominal p=0.03), as measured by estimated glomerular filtration rate, and a substantial proportion of patients achieved a reduction in C3c staining intensity (nominal p<0.0001). Pegcetacoplan showed favourable safety and tolerability, consistent with its established profile.

"There is an urgent need for an approved treatment for C3G and IC-MPGN that can prolong kidney function," said Jeffrey Eisele, Ph.D., Chief Development Officer at Apellis. The EMA validation leads us one step closer to potentially bringing this treatment to European patients in need. Additionally, we continue to advance the regulatory process in the U.S., with a potential launch of pegcetacoplan for C3G and primaryIC-MPGN in the second half of 2025, if approved."

Aspaveli first received a marketing authorisation from the European Commission in 2021 for the treatment of paroxysmal nocturnal haemoglobinuria.

About the VALIANT Study

The VALIANT Phase 3 study (NCT05067127) is a randomised, placebo-controlled, double-blinded, multi-center 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 primary IC-MPGN suffer from kidney failure within five to 10 years of diagnosis, requiring a burdensome kidney transplant or lifelong dialysis. Additionally, 90% of patients who previously received a kidney transplant will experience disease recurrence. The diseases are estimated to affect 5,000 people in the United States and up to 8,000 in Europe.

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About Pegcetacoplan in Rare Diseases
Pegcetacoplan is a targeted C3 and C3b 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 nephrology and haematology. 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-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 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 2024, revenue amounted to SEK 26 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. More about Sobi at sobi.com and LinkedIn.

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 many serious diseases. For more information, please visit apellis.com, or follow us on X (Twitter) and LinkedIn.

Contacts
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