

## PRESS RELEASE

Stockholm, Sweden, 15 December 2021

### Aspaveli® (pegcetacoplan) approved in EU as orphan drug for treatment of PNH

- *The first targeted C3 therapy approved in the EU*
- *Approval based on results from head-to-head PEGASUS phase 3 study where Aspaveli demonstrated superiority to eculizumab in improving haemoglobin levels<sup>1</sup>*
- *Aspaveli will have market exclusivity for paroxysmal nocturnal haemoglobinuria (PNH) based on orphan drug designation*

[Swedish Orphan Biovitrum AB \(publ\)](#) (Sobi™) (STO:SOBI) and Apellis Pharmaceuticals, Inc. (Nasdaq: APLS) announced today that the European Commission (EC) has approved Aspaveli® (pegcetacoplan), the first and only targeted C3 therapy, for the treatment of adults with paroxysmal nocturnal haemoglobinuria (PNH) who are anaemic after treatment with a C5 inhibitor for at least three months. Based on the recommendation from the European Medicines Agency's Committee for Orphan Medicinal Products, the EC determined that pegcetacoplan continues to meet the criteria for the orphan drug designation status granted in 2017 for the treatment of PNH.

PNH is a rare, chronic and life-threatening blood disorder where uncontrolled complement activation leads to the destruction of oxygen-carrying red blood cells through intravascular haemolysis and extravascular haemolysis. Characterised by persistently low haemoglobin, PNH can result in frequent transfusions and debilitating symptoms such as severe fatigue caused by anaemia. Despite improvements in haemolytic activity with C5 inhibitor treatment, approximately 72 per cent of people with PNH treated with C5 inhibitors remain anaemic, according to a retrospective and a cross-sectional study.<sup>2,3</sup>

"The European Commission's approval of Aspaveli is a milestone for people living with PNH across Europe," said, Guido Oelkers, CEO and President at Sobi. "The symptoms of PNH can significantly impact quality of life. In addition, despite current therapy, many people still require frequent blood transfusions. We are now working with EU member states to provide access to this important medicine as quickly as possible."

"As the first and only targeted C3 therapy in Europe, Aspaveli has the potential to elevate the standard of care for patients living with PNH," said Federico Grossi, MD, PhD, Chief Medical Officer of Apellis. "Today's approval represents the first new class of complement medicines in Europe in over a decade, building on the launch of this important treatment in the United States."

The approval is based on the results from the head-to-head PEGASUS phase 3 study, which evaluated the efficacy and safety of Aspaveli compared to eculizumab at 16 weeks in adults with PNH who had persistent anaemia despite treatment with eculizumab. The full safety and efficacy results were published in [The New England Journal of Medicine](#) in March 2021.<sup>1</sup>



Orphan drug designation is granted to therapies that treat a serious disease that affects fewer than five in 10,000 people in the EU and provide a significant benefit over existing treatments. Aspaveli will have market exclusivity based on orphan drug designation for PNH.

#### **About Aspaveli®/Empaveli™**

Aspaveli/Empaveli (pegcetacoplan) is a targeted C3 therapy designed to regulate excessive activation of the complement cascade, part of the body's immune system, which can lead to the onset and progression of many serious diseases. Aspaveli is approved in the EU as an orphan drug for the treatment of adults with paroxysmal nocturnal haemoglobinuria (PNH) who are anaemic after treatment with a C5 inhibitor for at least three months and in the United States as Empaveli for the treatment of adults with PNH. The therapy is also under investigation for several other rare diseases across haematology, nephrology, and neurology.

#### **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 targeted C3 therapies, Apellis aim to develop transformative therapies for a broad range of debilitating diseases that are driven by excessive activation of the complement cascade, including those within haematology, ophthalmology, nephrology, and neurology. For more information, please visit <http://apellis.com>.

#### **About Sobi™**

Sobi is a specialised international biopharmaceutical company transforming the lives of people with rare diseases. Sobi is providing sustainable access to innovative therapies in the areas of haematology, immunology and specialty indications. Today, Sobi employs approximately 1,500 people across Europe, North America, Middle East and Asia. In 2020, Sobi's revenues amounted to SEK 15.3 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. More about Sobi at [sobi.com](https://sobi.com), [LinkedIn](#) and [YouTube](#).

#### **Contacts Sobi**

To contact the Sobi Investor Relations Team, [click here](#). For Sobi Media contacts, [click here](#).

#### **Apellis**

Media:  
Lissa Pavluk  
[media@apellis.com](mailto:media@apellis.com)  
+1.617.977.6764

Investors:  
Meredith Kaya  
[meredith.kaya@apellis.com](mailto:meredith.kaya@apellis.com)  
+1.617.599.8178

## Reference

1. Hillmen P, Szer J, Weitz I, et al. Pegcetacoplan versus Eculizumab in Paroxysmal Nocturnal Hemoglobinuria. N Engl J Med. DOI: 10.1056/NEJMoa2029073.
2. McKinley C. Extravascular Hemolysis Due to C3-Loading in Patients with PNH Treated with Eculizumab: Defining the Clinical Syndrome. Blood. 2017;130:3471.
3. Dingli ASH 2020 Abstract/ p.1/ Methods/ ln.1-2; p.2/ Results/ln.7-9; ln.14-1.
4. Risitano et al. 2009.