# PRESS RELEASE

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## Aspaveli® (pegcetacoplan) receives positive CHMP opinion for treatment of PNH

Swedish Orphan Biovitrum AB (publ) (Sobi™) (STO:SOBI) and Apellis Pharmaceuticals, Inc. (Nasdaq: APLS) announce today that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency has adopted a positive opinion recommending the marketing authorisation of Aspaveli® (pegcetacoplan) for the treatment of adults with paroxysmal nocturnal haemoglobinuria (PNH) who are anaemic after treatment with a C5 inhibitor for at least three months. The positive opinion from the CHMP is now referred to the European Commission for an approval decision.

PNH is a rare, chronic, life-threatening blood disorder where uncontrolled complement activation leads to the destruction of oxygen-carrying red blood cells through intravascular and extravascular haemolysis. Characterised by persistently low haemoglobin, PNH can result in frequent transfusions and debilitating symptoms such as severe fatigue. Despite improvements in haemolytic activity with C5 inhibitor treatment, approximately 72 per cent of C5-treated patients remain anaemic, according to a retrospective and a cross-sectional study.[1,2]

“Today’s positive opinion by the CHMP is a significant milestone for people living with PNH across Europe,” said Ravi Rao, Head of Research & Development and Chief Medical Officer at Sobi. “We hope to make a difference to the lives of people living with rare diseases, and if Aspaveli is approved by the European Commission, it will offer patients and treating physicians a new class of complement medicines for the treatment of PNH.”

The positive opinion is based on the results from the head-to-head phase 3 PEGASUS 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 results were published in The New England Journal of Medicine in March 2021.[2]

“Building on our recent U.S. approval, the positive CHMP opinion moves us one step closer towards bringing this important treatment to patients across Europe,” said Federico Grossi, MD, PhD, Chief Medical Officer at Apellis. “If approved, Aspaveli has the potential to redefine treatment for patients with PNH, so we look forward to the European Commission’s final decision.”

Aspaveli is the European trade name for pegcetacoplan, which is known as Empaveli™ in the United States where it is approved for the treatment of adults with PNH.

**About the PEGASUS study**

PEGASUS (APL2-302; NCT03500549) was a multi-centre, randomised, head-to-head phase 3 study in 80 adults with paroxysmal nocturnal haemoglobinuria (PNH). The primary objective of this study was to establish the efficacy and safety of Aspaveli®/Empaveli™ (pegcetacoplan) compared to eculizumab.

**About Aspaveli®/Empaveli™ (pegcetacoplan)**
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. Empaveli is approved in the United States for the treatment of adults with paroxysmal nocturnal haemoglobinuria (PNH). The Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency has adopted a positive opinion for Aspaveli for the treatment of adults with PNH who are anaemic after treatment with a C5 inhibitor for at least three months. The positive opinion from the CHMP is now referred to the European Commission for an approval decision. The therapy is also under investigation for several other rare diseases across haematology, nephrology, and neurology.

**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 aims 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. You can find more information about Sobi at sobi.com.

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## Contacts


Sobi

Thomas Kudsk Larsen
Head of Communication and Investor Relations

Maria Kruse
Director of Communication and Investor Relations

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

Apellis

Investors:
Meredith Kaya
meredith.kaya@apellis.com
+1 617 599 8178

Media:
Lissa Pavluk
media@apellis.com
+1 617 977 6764


**Notes**

1. McKinley C. Extravascular Hemolysis Due to C3-Loading in Patients with PNH Treated with Eculizumab: Defining the Clinical Syndrome. Blood. 2017;130:3471.
2. Dingli ASH 2020 Abstract/ p.1/ Methods/ ln.1-2; p.2/ Results/ln.7-9; ln.14-15.
3. Hillmen P, Szer J, Weitz I, et al. Pegcetacoplan versus Eculizumab in Paroxysmal Nocturnal Hemoglobinuria. N Engl J Med. DOI: 10.1056/NEJMoa2029073.