PRESS RELEASE

Stockholm, Sweden, 8 May 2024

Aspaveli® (pegcetacoplan) approved in Europe for use among treatment naïve adult patients with PNH

Sobi® today announced that the European Commission (EC) has approved an indication extension for Aspaveli® (pegcetacoplan) for treatment of adult patients with paroxysmal nocturnal haemoglobinuria (PNH) who have haemolytic anaemia.

Aspaveli is already approved in Europe for the treatment of adults with PNH who are anaemic after treatment with a C5 inhibitor for at least three months. Aspaveli is now the first C3 inhibitor approved for first-line treatment of PNH in Europe, offering effective outcomes by improving haemoglobin and other clinical markers due to its unique mode of action.

“Today’s approval underscores the robust clinical data supporting Aspaveli 's efficacy and safety profile, offering healthcare professionals and patients an expanded toolkit for effectively managing PNH,” said Lydia Abad-Franch MD, Head of R&D and Medical Affairs, and Chief Medical Officer at Sobi. “European patients will now be able to initiate treatment with Aspaveli at diagnosis or switch from their current C5 inhibitor treatment if they present indicators of haemolytic anaemia. This important milestone reflects our dedication to improving treatment options for those affected by this rare and complex condition.”

PNH is a rare, chronic and life-threatening blood disorder where uncontrolled complement activation leads to the destruction of oxygen-carrying red blood cells. Characterised by persistently low haemoglobin, PNH can result in frequent transfusions and debilitating symptoms such as severe fatigue caused by anaemia. Despite improvements with C5 inhibitor treatment, up to 86 per cent of people with PNH treated with C5 inhibitors remain anaemic, according to cross-sectional surveys conducted in US and EU.[1,2, 3,]

The indication extension is based on data from APL2-308 (PRINCE, NCT04085601), an open-label, randomised, comparator-controlled study that enrolled patients with PNH who had not been treated with any complement inhibitor within three months prior to enrolment and with haemoglobin levels less than the lower limit of normal and lactate dehydrogenase levels ≥1.5 times the upper limit of normal. Efficacy and safety of Aspaveli was evaluated over a duration of 26 weeks compared to standard of care (e.g., transfusions, corticosteroids, supplements such as iron, folate, and vitamin B12; excluding complement inhibitors).[4,5.]

Aspaveli is the European trade name for pegcetacoplan, which is known as Empaveli® in the United States where it is also approved for the treatment of adults with PNH and commercialised by Apellis.

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. Pegcetacoplan is approved for the treatment of paroxysmal nocturnal haemoglobinuria (PNH) as Aspaveli/Empaveli in the European Union, United States, and other countries globally. Aspaveli/Empaveli is also under investigation for other rare diseases across haematology and nephrology.

Swedish Orphan Biovitrum AB (publ) (Sobi®)
Postal address SE-112 76 Stockholm, Sweden
Phone: +46 8 697 20 00 | www.sobi.com

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 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.

Contacts
To contact the Sobi Investor Relations Team, please click here. For Sobi Media contacts, click here.

References

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