14 October 2021
EMA/CHMP/555115/2021
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Aspaveli

pegcetacoplan

On 14 October 2021, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Aspaveli,[2] intended for the treatment of adult patients with paroxysmal nocturnal haemoglobinuria.

The applicant for this medicinal product is Swedish Orphan Biovitrum AB (publ).

Aspaveli will be available as a 1,080 mg solution for infusion. The active substance of Aspaveli is pegcetacoplan, a selective immunosupressant (ATC code: L04AA54). Pegcetacoplan binds to complement protein C3 and its activation fragment C3b with high affinity, thereby regulating the cleavage of C3 and the generation of downstream effectors of complement activation.

The benefits of Aspaveli are its ability to improve haemoglobin levels and to avoid transfusions in patients with paroxysmal nocturnal haemoglobinuria who are anaemic after treatment with a C5 inhibitor. The most common side effects are upper respiratory tract infection, headache, abdominal pain, diarrhoea, injection site reactions, fatigue and pyrexia.

The full indication is:

> ASPAVELI is indicated in the treatment of adult patients with paroxysmal nocturnal haemoglobinuria (PNH) who are anaemic after treatment with a C5 inhibitor for at least 3 months.
>
> ASPAVELI should be prescribed by physicians experienced in the management of patients with haematological disorders.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained

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