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Aspaveli (*pegcetacoplan*)

An overview of Aspaveli and why it is authorised in the EU

What is Aspaveli and what is it used for?

Aspaveli is a medicine to treat adults with paroxysmal nocturnal haemoglobinuria (PNH), a condition in which there is excessive breakdown of red blood cells (haemolysis), leading to large amounts of haemoglobin (the protein in red blood cells that carries oxygen around the body) being released into the urine. Aspaveli is used in patients who continue to have anaemia (low levels of red blood cells) despite treatment with a type of medicine called a C5 inhibitor for at least 3 months.

Paroxysmal nocturnal haemoglobinuria is rare, and Aspaveli was designated an 'orphan medicine' (a medicine used in rare diseases) on 22 May 2017. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu3171873.

Aspaveli contains the active substance pegcetacoplan.

How is Aspaveli used?

The medicine can only be obtained with a prescription. Treatment should be started under the supervision of a healthcare professional experienced in the management of blood-related disorders.

Aspaveli is given as an infusion (drip) under the skin in the belly, thigh or upper arms. It is given twice a week (on day 1 and 4).

Patients should continue to received their C5 inhibitor for 4 weeks after starting Aspaveli before stopping the C5 inhibitor..

Patients can give themselves the drip once they have been trained to do so.

For more information about using Aspaveli, see the package leaflet or contact your doctor or pharmacist.

How does Aspaveli work?

Aspaveli is made of two synthetic peptides (short chains of amino acids) linked together, which target and attach to the C3 complement protein, which is a part of the body's defence system called the 'complement system'.

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In patients with PNH, the complement proteins are over-active and damage the patients' own cells. By blocking the C3 complement protein, Aspaveli prevents complement proteins from damaging cells, thereby helping to relieve the symptoms of this disease.

What benefits of Aspaveli have been shown in studies?

Aspaveli was shown to be effective at preventing breakdown of haemoglobin and increasing its blood levels in patients with PNH who had been treated with eculizumab for at least 3 months but were still anaemic.

The main study was conducted in 80 patients with PNH currently being treated with eculizumab, a C5 inhibitor, but who continued to be anaemic (haemoglobin level <10.5 g/dL) despite this treatment. Patients were either switched to Aspaveli or continued their eculizumab treatment. After 16 weeks, the haemoglobin levels in patients receiving Aspaveli increased on average by 2.37 g/dl while it decreased by 1.47 g/dl on average in patients who were still treated with eculizumab. During this period, 6 of 41 people given Aspaveli needed a blood transfusion, compared with 33 of 39 treated with eculizumab.

What are the risks associated with Aspaveli?

The most common side effects with Aspaveli (which may affect more than 1 in 10 people) are injection site reactions (reddening of the skin, itching, swelling and site pain), upper respiratory tract infection, abdominal pain, diarrhoea, headache, tiredness, and fever. The most serious side effects are haemolysis (breakdown of red blood cells) and thrombocytopenia (low levels of blood platelets), which may affect up to 1 in 10 people.

Based on its mechanism of action, Aspaveli may increase the risk of infections. Aspaveli must not be used in patients with an ongoing infection caused by certain bacteria known as encapsulated bacteria including *Neisseria meningitidis*, *Streptococcus pneumoniae*, and *Haemophilus influenzae*. It must also not be used in patients who are not currently vaccinated against infection by these bacteria. People who get vaccinated must take appropriate antibiotics to reduce the risk of infection for two weeks after vaccination.

For the full list of side effects and restrictions, see the package leaflet.

Why is Aspaveli authorised in the EU?

Aspaveli has been shown to be effective at increasing blood haemoglobin levels in patients with PNH who had been treated with eculizumab for at least 3 months but were still anaemic. It also reduced the need for blood transfusions in these patients. In terms of safety, although the data on safety are limited due to the small number of patients included in the main study, the side effects of Aspaveli are considered manageable, considering the risk minimisation measures in place.

The European Medicines Agency decided that Aspaveli's benefits are greater than its risks and it can be authorised for use in the EU.

What measures are being taken to ensure the safe and effective use of Aspaveli?

The company that markets Aspaveli will ensure that distribution of the medicine occurs only after checking that the patient has been vaccinated appropriately. The company will also provide prescribers and patients with information on the safety of the medicine, and will send reminders to prescribers and pharmacists to check if any further vaccination is needed for patients taking Aspaveli. Patients will also

be given a special card that explains the symptoms of certain types of infection, instructing patients to seek medical care immediately if they experience them.

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Aspaveli have also been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Aspaveli are continuously monitored. Suspected side effects reported with Aspaveli are carefully evaluated and any necessary action taken to protect patients.

Other information about Aspaveli

Aspaveli received a marketing authorisation valid throughout the EU on 13 December 2021.

Further information on Aspaveli can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/aspaveli.

This overview was last updated in 12-2021.