EMADOC-1829012207-38751
EMEA/H/C/005553

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 used to treat:

- adults with paroxysmal nocturnal haemoglobinuria (PNH), an acquired disease 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 with PNH who have anaemia (low levels of red blood cells) due to haemolysis;
- adults and adolescents aged 12 to 17 years with complement 3 glomerulopathy (C3G) or primary immune-complex membranoproliferative glomerulonephritis (IC-MPGN), either in combination with a RAS inhibitor (a medicine that acts on the renin-angiotensin system) or alone in patients who cannot take a RAS inhibitor. C3G and IC-MPGN are diseases in which the immune system mistakenly damages the kidney's filtering units; this prevents the kidneys from filtering the blood correctly, leading to toxin build-up, reduced urine production and swelling.

PNH, C3G and IC-MPGN are rare, and Aspaveli was designated an 'orphan medicine' (a medicine used in rare diseases). Further information on the orphan designations can be found on the EMA website (PNH: 22 May 2017; C3G with or without immune complexes: 10 November 2022).

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 or kidney disorders.

Aspaveli is given as an infusion under the skin of the belly, thighs, hips or upper arms twice a week (on days 1 and 4). Patients may give themselves the infusion if their doctor considers it appropriate and they have been trained to do so. Unless there is a clinical reason for stopping treatment, Aspaveli is continued for life.

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

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How does Aspaveli work?

The active substance in Aspaveli, pegcetacoplan, is made of two synthetic peptides (short chains of amino acids) that are linked together. It attaches to the C3 complement protein, which is a part of the immune system (the body's natural defences) called the 'complement system'.

In people with PNH, the complement proteins are overactive and damage the patients' own blood cells. By blocking the C3 complement protein, Aspaveli prevents complement proteins from damaging blood cells, thereby helping to relieve the symptoms of PNH.

In people with C3G and primary IC-MPGN, breakdown products of the C3 complement protein build up in the kidneys, damaging them and leading to decreased kidney function. By blocking the C3 complement protein, Aspaveli reduces the build-up of C3 breakdown products in the kidneys, thereby preventing further kidney damage.

What benefits of Aspaveli have been shown in studies?

Paroxysmal nocturnal haemoglobinuria (PNH)

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

The study involved 80 adults with PNH being treated with eculizumab, a medicine known as a complement inhibitor, but who were still anaemic (haemoglobin level <10.5 g/dL) despite this treatment. Patients were either switched to Aspaveli or continued their eculizumab treatment. After 16 weeks, haemoglobin levels in patients receiving Aspaveli increased on average by 2.37 g/dL while they decreased on average by 1.47 g/dL in patients who were still treated with eculizumab. During this period, 6 of 41 patients given Aspaveli needed a blood transfusion, compared with 33 of 39 treated with eculizumab.

A second study evaluated the use of Aspaveli in 53 adults with PNH who had not received a complement inhibitor in the 3 months leading up to the study. Aspaveli was more effective than supportive care (treatment to prevent or relieve the symptoms of the disease) in controlling the breakdown of red blood cells and stabilising haemoglobin levels. After 26 weeks of treatment, haemoglobin levels had stabilised (meaning they did not decrease by more than 1 g/dL without the patient having a blood transfusion) in around 86% of patients given Aspaveli (30 out of 35) compared to none of those who received supportive care (0 out of 18).

The study also evaluated the effect of treatment on blood levels of lactate dehydrogenase (LDH), a marker for tissue damage that increases when red blood cells break down. After 26 weeks of treatment, patients given Aspaveli had an average decrease of 1,870 units/L in their LDH levels compared with an average decrease of 400 units/L in those who received supportive care. During this period, around 91% of those given Aspaveli (32 out of 35) did not require a transfusion, compared with 6% of those given supportive care (1 out of 18).

Complement 3 glomerulopathy (C3G) and primary immune-complex membranoproliferative glomerulonephritis (IC-MPGN)

Aspaveli was shown to be more effective than placebo (a dummy treatment) at reducing kidney damage in adults and adolescents from 12 years of age with C3G or primary IC-MPGN.

In a main study involving 124 adults and adolescents from 12 years of age with C3G or IC-MPGN, participants were given either Aspaveli or placebo twice a week. The main measure of effectiveness was a reduction in proteinuria (protein in the urine). As healthy kidneys keep nearly all proteins in the

blood, the presence of proteins in urine indicates kidney damage. After 26 weeks of treatment, people receiving Aspaveli had an approximately 68% reduction in their urine protein levels compared with those given placebo. This effect was maintained after 1 year of treatment.

What are the risks associated with Aspaveli?

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

In people with PNH, the most common side effects with Aspaveli (which may affect more than 1 in 10 people) include reactions at the infusion site (reddening of the skin, itching, swelling, bruising and pain), upper respiratory tract (nose and throat) infection, abdominal (belly) pain, diarrhoea, haemolysis, headache, tiredness, fever, cough, urinary tract infection (infection of the parts of the body that collect and pass out urine), pain in extremity (arm or leg), dizziness, joint pain, back pain and complications with vaccination. Some side effects can be serious. The most frequent serious side effects include haemolysis (which may affect more than 1 in 10 people) and sepsis (blood poisoning; which may affect up to 1 in 10 people).

In people with C3G or primary IC-MPGN, the most common side effects with Aspaveli (which may affect more than 1 in 10 in people) include reactions at the infusion site and upper respiratory tract infections. The most frequent serious side effects include acute kidney injury (sudden damage to the kidneys that causes them to not work properly) and pneumonia (infection of the lungs).

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 these bacteria unless they take appropriate antibiotics to reduce the risk of infection for two weeks after vaccination.

Why is Aspaveli authorised in the EU?

Aspaveli is effective at increasing blood haemoglobin levels in adults with PNH who had been treated with eculizumab for at least 3 months but still had anaemia. Aspaveli is also more effective than supportive care at stabilising haemoglobin levels and controlling the breakdown of red blood cells in adults with PNH who had not been treated with complement inhibitors for at least 3 months. It also reduces the need for blood transfusions in adults with PNH. However, uncertainties related to the design of the study in patients who had not been treated with complement inhibitors for at least 3 months limited the evaluation of the benefits and risks of Aspaveli in these patients.

Aspaveli is also effective at reducing proteinuria in adults and adolescents from 12 years of age with C3G or primary IC-MPGN, which suggests reduced kidney damage. Although a reduction in proteinuria has not been fully proven to predict long-term kidney function, the available data were considered sufficient to demonstrate the effectiveness of Aspaveli in adults and adolescents from 12 years of age with C3G or primary IC-MPGN.

In terms of safety, although the data on safety are limited due to the small number of patients in the main studies, the side effects of Aspaveli are considered manageable with the measures in place to minimise its risks.

The European Medicines Agency therefore 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 01-2026.