

# Medicine: pegcetacoplan (brand name: Aspaveli®)

Swedish Orphan Biovitrum Ltd

The Scottish Medicines Consortium (SMC) has assessed pegcetacoplan for the treatment of adults with paroxysmal nocturnal haemoglobinuria (PNH). It is used to treat patients who are anaemic (have low levels of red blood cells) after at least 3 months of treatment with a C5 inhibitor (a type of medicine used to treat PNH, e.g. eculizumab or ravulizumab). This document summarises the SMC decision and what it means for patients.

## What has SMC said?

After careful consideration, SMC has accepted pegcetacoplan for the treatment of PNH as described above in certain patients (restricted use). The restriction means that the patients must be being treated under the advice of the National PNH Service.

This SMC advice takes into account a confidential discount offered by the pharmaceutical company that improves the cost-effectiveness of pegcetacoplan.

### What does SMC's decision mean for patients?

If your healthcare professional thinks that pegcetacoplan for use as described above is the right medicine for you, you should be able to have the treatment on the NHS in Scotland.



## What is pegcetacoplan used for?

Pegcetacoplan is used for the treatment of PNH, a potentially life-threatening condition where the body breaks down too many red blood cells. This can lead to anaemia (low red blood cell counts), tiredness, difficulty in functioning, pain, dark urine, shortness of breath, difficulty swallowing, erectile dysfunction and blood clots. It is used to treat patients who have anaemia despite 3 months or more of treatment with a C5 inhibitor such as eculizumab or ravulizumab. Patients must be being treated under the advice of the National PNH Service.

## How does pegcetacoplan work?

In patients with PNH the complement system (part of the body's immune system) is overactive and attacks the body's red blood cells. Pegcetacoplan binds to a protein in the complement system called C3. By binding to C3 pegcetacoplan blocks its effects and helps to prevent the destruction of red blood cells that causes the symptoms of PNH.

## How does SMC make its decision?

SMC carefully considers every new medicine to make sure it benefits patients and is considered to be an acceptable use of the limited resources in NHSScotland.

To do this SMC considers the following:

- Evidence from the company about how well the medicine works compared with current treatments available in Scotland, in relation to how much they will cost to buy and administer.
- Information from patient groups about the potential impact of the medicine on patients and carers.
- Advice from healthcare professionals about any benefits of the new medicine compared to current treatment, along with how the new medicine is likely to be used.

When SMC assesses a medicine it takes account of the needs of all patients in NHSScotland, not only those who may be treated with the medicine under consideration.

You can find more detailed information about the SMC assessment of pegcetacoplan by looking at the SMC Detailed Advice Document (SMC2451).

## More information

The organisation below can provide more information and support for people with PNH and their families. SMC is not responsible for the content of any information provided by external organisations.

### PNH Scotland



<https://pnhscotland.org.uk>



07967 118 762

You can find out more about pegcetacoplan (Aspaveli®) in the Patient Leaflet by searching for the medicine name on the electronic medicines compendium (EMC) website.



<https://www.medicines.org.uk/emc/>