

NCPE Assessment

Plain English Summary

April 2024

Drug name: Pegcetacoplan
(pronounced peg-CET-ah-co-plan) for the treatment of paroxysmal nocturnal haemoglobinuria in adult patients who are anaemic after treatment with a C5 inhibitor for at least three months.

Brand name: Aspaveli®

HTA ID: 21064

What is the NCPE?

The National Centre for Pharmacoeconomics (NCPE) is a team of experts who look at the health benefits and costs of medicines. The HSE asks us to advise on whether or not a new medicine is good value for money. We give unbiased advice to help the HSE provide the most effective, safe and cost-effective (value for money) treatments for patients.

How do we make our recommendations?

Our main focus is on the health benefits and cost effectiveness of a medicine. We look at the wider costs and health benefits associated with a new medicine, for example:

- Does the new medicine work better than other treatments available in Ireland?
- Is the new medicine easier to give or easier to take compared with other treatments available in Ireland?
- Does the new medicine reduce the need for patients to be hospitalised?
- Does the new medicine improve the quality of a patient's life over other treatments available in Ireland?
- Will the new medicine save resources elsewhere within the health system?

We review the information from clinical trials along with the cost and value for money data presented by the pharmaceutical company. We ask doctors and other healthcare professionals for advice about any health benefits of the new medicine compared with current treatments. We also ask patient organisations to send us their views on how the new drug may improve patients' day-to-day experience of living with a disease.

What is pegcetacoplan used for?

Pegcetacoplan 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 being released into the urine. Pegcetacoplan 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 three months.

What recommendation has the NCPE made to the HSE?

We have recommended that the HSE should consider not funding pegcetacoplan unless its cost effectiveness (value for money) can be improved. The HSE will consider our recommendation and make the final decision about reimbursement (funding). When making the funding decision, the HSE will also consider the additional [criteria](#) outlined in the Health (Pricing and Supply of Medical Goods) Act 2013.

We have received a Patient Organisation Submission from PNH Support about pegcetacoplan and shared it with the HSE. This submission will form part of the data that the HSE considers.

Why did we make this recommendation?

After reviewing the data presented by the pharmaceutical company, we recommended that the HSE consider not funding pegcetacoplan unless the HSE can agree a suitable price reduction with the pharmaceutical company. This is because we believe pegcetacoplan may work as well or better than other ways to manage this condition. However, the price of pegcetacoplan is too high compared with other ways to manage this condition, and we believe that pegcetacoplan is very poor value for money.

Next steps

When the HSE receives our recommendation, it will look at all the relevant data about pegcetacoplan. The HSE makes the final decision on reimbursement.

Where can I get more information?

You can get more information about pegcetacoplan from the following online options:

- the NCPE Technical Summary Document
- Aspaveli® European Public Assessment Report (EPAR) – [Medicine Overview](#)
or,
- searching for pegcetacoplan on our website (www.ncpe.ie);
- searching for pegcetacoplan on the European Medicines Agency (EMA) website

(www.ema.europa.eu).

Please refer to the NCPE website for updated information on the reimbursement status of this medicine