



NCPE Plain English Summary

Drug name: Pegvaliase (*pronounced: peg val' i ase*) for the treatment of adults and adolescents with phenylketonuria aged from 16 years.

Brand name: Palynziq®

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 pegvaliase used for?

Pegvaliase is a medicine that is used to treat phenylketonuria (PKU) in adults and adolescents from 16 years of age. Patients with this genetic disease cannot process the amino acid phenylalanine from dietary protein, and as a result the amino acid builds up in the blood to abnormally high levels, causing problems in the nervous system. Pegvaliase is used in patients whose blood levels of phenylalanine have not been adequately controlled with other treatments.

What recommendation has the NCPE made to the HSE?

We have recommended that the HSE should consider funding pegvaliase if 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 the PKU Association of Ireland about pegvaliase 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 concluded that pegvaliase be considered for reimbursement if cost effectiveness can be improved relative to existing treatments. We have completed a full health technology assessment for this medicine. We recommend that the HSE consider providing this medicine if the HSE can agree a suitable price reduction with the pharmaceutical company. We believe the medicine may work as well or better than other ways to manage this condition. However, the price of the medicine is too high compared with other ways to manage this condition, and we believe that the medicine is not value for money.

The HSE considers a number of factors along with our recommendation when deciding whether to provide this medicine. These factors are listed in the Health (Pricing and Supply of Medical Goods) Act 2013.

Next steps

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

Where can I get more information?

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

- the NCPE Technical Summary Document
- Pegvaliase European Public Assessment Report (EPAR) – [Summary for the public](#)
or
- searching for pegvaliase on our website (www.ncpe.ie);
- searching for pegvaliase 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.

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