



NCPE Plain English Summary

Drug name: Voretigene neparvovec (*pronounced: voe RET i jeen ne PAR voe vek*) for the treatment of adult and paediatric patients with vision loss due to inherited retinal dystrophy caused by confirmed biallelic *RPE65* mutations and who have sufficient viable retinal cells.

Brand name: Luxturna®

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 voretigene neparvovec used for?

Voretigene neparvovec is used to treat adults and children with loss of vision due to inherited retinal dystrophy which is a rare genetic disorder of the retina (the light sensitive membrane at the back of the eye). It works by delivering genes into the body and is a type of advanced

therapy medicine. Voretigene neparvovec can only be used by patients who still have enough working cells left in the retina, and when the disease is caused by mutations (changes) in the gene *RPE65*.

What recommendation has the NCPE made to the HSE?

We have recommended that the HSE should consider not funding voretigene neparvovec 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 Fighting Blindness about voretigene neparvovec 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 recommend that the HSE consider not providing voretigene neparvovec unless the HSE can agree a suitable price reduction with the pharmaceutical company. This is because 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 very poor value for money.

Next steps

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

Where can I get more information?

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

- the NCPE Technical Summary Document
- Luxturna[®] European Public Assessment Report (EPAR) – [Medicine Overview](#)
- searching for voretigene neparvovec on our website (www.ncpe.ie);
- searching for voretigene neparvovec 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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