



NCPE Plain English Summary

Drug name: Idebenone (*pronounced: eye-deb-eh-known*) for the treatment of Leber's Hereditary Optic Neuropathy (LHON).

Brand name: Raxone®

What is the NCPE?

The National Centre for Pharmacoeconomics (NCPE) is a team of experts who look at the 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 clinical benefits and cost-effectiveness of a medicine. We look at the wider costs and 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 all 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 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 idebenone used for?

Idebenone is used to treat sight loss in adults and teenagers with Leber's Hereditary Optic Neuropathy (LHON). LHON is a rare genetic disease that causes sight loss.

What recommendation has the NCPE made to the HSE?

We have recommended that the HSE should consider not funding idebenone unless its cost-effectiveness 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 Ireland about idebenone 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 there is not enough robust evidence to show that this medicine is a clinically effective treatment. We also believe this medicine does not show value for money at the present price when compared with other medicines available to treat this condition. We recommend that the HSE should only consider funding this medicine if it can agree a price discount with the pharmaceutical company.

Next steps

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

Where can I get more information?

You can get more information about idebenone from one of the following online options:

- the [NCPE technical summary](#),
- Raxone® European Public Assessment Report (EPAR) – [Summary for the public](#), or
- searching for the medicine name on our website (www.ncpe.ie),
- searching for the medicine name 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.

Date Published: October 2019