



NCPE Plain English Summary

Drug name: Patisiran for the treatment of hereditary transthyretin-mediated (hATTR) amyloidosis. **Brand name:** Onpattro®

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 Patisiran used for?

hATTR amyloidosis is a very rare life threatening genetic disease caused by mutations (genetic changes) in the transthyretin (TTR) gene. This leads to the production of abnormal TTR protein which causes build-ups called amyloid deposits in the body's tissues and organs (referred to as amyloidosis). If the amyloid deposits build up around the nerves this causes nerve damage (polyneuropathy). Patisiran is used to treat adult patients in the first two stages of nerve damage due to hATTR (stage 1, when the patient is able to walk unaided, and stage 2, when the patient can still walk but needs help).

What recommendation has the NCPE made to the HSE?

We have recommended that the HSE should consider not funding patisiran 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.

Why did we make this recommendation?

After reviewing the data presented by the pharmaceutical company, we concluded that although we believe the medicine may work, the price of the medicine is too high compared with other ways to manage this condition, and therefore we believe that the medicine is very poor value for money. We recommend that the HSE consider **not** providing this medicine unless the HSE can agree a suitable price reduction with the pharmaceutical company.

Next steps

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

Where can I get more information?

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

- the NCPE Technical Summary Document

- Onpattro® European Public Assessment Report (EPAR) – [Summary for the public](#)
or
- searching for patisiran on our website (www.ncpe.ie);
- searching for patisiran 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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