



NCPE Plain English Summary

Drug name: Inotersen (*pronounced: Ino ter sen*) for the treatment of stage 1 or stage 2 polyneuropathy in adults with hereditary transthyretin amyloidosis

Brand name: Tegsedi®

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 inotersen used for?

Inotersen is used to treat adult patients with hereditary transthyretin amyloidosis who present with stage 1 or stage 2 polyneuropathy which affects nerve function. Sensory symptoms include pain, numbness and tingling sensations and motor symptoms include a loss of power in muscle groups eventually leading to patients being wheelchair bound. Other nervous system effects (autonomic effects) include dizziness or fainting, nausea, vomiting, severe

diarrhoea and/or constipation. These symptoms result from the accumulation of amyloid protein in the nerves and inotersen, which is administered subcutaneously once weekly, inhibits the production of this protein resulting in a significant improvement in neurological disease progression and quality of life.

Patisiran is another drug which reduces amyloid protein and is also licensed for use in stage 1 and stage 2 polyneuropathy in adults with hereditary transthyretin amyloidosis. It is administered intravenously every three weeks and has been available in Ireland since October 2021.

What recommendation has the NCPE made to the HSE?

We have recommended that the HSE should consider funding inotersen for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis 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 ATTR Amyloidosis Ireland Support Group about inotersen 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 considers providing inotersen. This is because we believe inotersen may work as well or better than other ways to manage this condition.

Next steps

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

Where can I get more information?

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

- the NCPE Technical Summary Document
- Searching for inotersen on our website (www.ncpe.ie);
- searching for inotersen 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: February 2022