NCPE Assessment

Plain English Summary

December, 2025

Drug name: Omaveloxolone (pronounced oh-MA-vel-ox-oh-lone) for the treatment of Friedreich's ataxia in adults and adolescents aged 16 years and older.

Brand name: Skyclarys®

HTA ID: 24033

NCPE
National Centre for Pharmacoeconomics, Ireland

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 omaveloxolone used for?

Omaveloxolone is a medicine used in patients aged 16 years and older with Friedreich's ataxia, a rare inherited disease that causes damage to the nervous system, resulting in difficulties with coordination, balance and movement, fatigue, difficulty speaking, as well as an increased risk of cardiomyopathy (damage to the heart muscle) and diabetes.

What recommendation has the NCPE made to the HSE?

We have recommended that the HSE should consider not funding omaveloxolone. 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 Patient Organisation Submission from Friedreich's Ataxia Research Alliance (FARA) Ireland and Neuroataxia CLG t/a Ataxia Foundation Ireland about omaveloxolone 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 omaveloxolone. This is because we are unsure based on the available clinical evidence that omaveloxolone leads to meaningful improvements in Friedreich's ataxia symptoms. The current price of the medicine is too high, and there is no price at which omaveloxolone can be cost effective. 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 omaveloxolone. The HSE makes the final decision on reimbursement.

Where can I get more information?

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

- the NCPE Technical Summary Document
- Skyclarys® European Public Assessment Report (EPAR) – Skyclarys (omaveloxolone) Medicine Overview
- searching for omaveloxolone on our website (www.ncpe.ie);
- searching for omaveloxolone 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