National Centre for Pharmacoeconomics
NCPE Ireland

NCPE Plain English Summary

Drug name: Risdiplam *(pronounced: ris-di-plam*) for the treatment of spinal muscular atrophy

Brand name: Evrysdi®

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 risdiplam used for?

Risdiplam is a medicine used to treat patients from 2 months old with 5q spinal muscular atrophy (SMA), a genetic disease that causes weakness and wasting of the muscles including the lung muscles. It is intended for patients with SMA type 1, type 2 or type 3, or those who have up to 4 copies of a gene known as SMN2.

What recommendation has the NCPE made to the HSE?

We have recommended that the HSE should consider not funding risdiplam 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 SMA Ireland about risdiplam 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 believe it is not clear that the medicine works as well or better than other ways to manage this condition. 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 risdplam. The HSE makes the final decision on reimbursement.

Where can I get more information?

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

- the NCPE Technical Summary Document
  Risdplam European Public Assessment Report (EPAR) – Evrysdi, medicine overview, or
- searching for risdiplam on our website (www.ncpe.ie);
- searching for risdiplam 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:** March 2022