



NCPE Plain English Summary

Drug name: onasemnogene abeparvovec (*pronounced: on-ah-zem-no-gene ah-beh-par-voh-vec*) for the treatment of spinal muscular atrophy.

Brand name: Zolgensma®

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 onasemnogene abeparvovec used for?

Spinal muscular atrophy is a serious condition of the nerves that causes muscle wasting and weakness. Onasemnogene abeparvovec is a gene therapy medicine for treating spinal muscular atrophy in patients with inherited mutations affecting genes known as SMN1, who have either been diagnosed with SMA type 1 or have up to 3 copies of another gene known as SMN2.

What recommendation has the NCPE made to the HSE?

We have recommended that the HSE should consider not funding onasemnogene abeparvovec 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.

This assessment was undertaken as part of the BENELUXA collaboration. It is a joint assessment between Ireland, Netherlands and Belgium with Austria acting as reviewer. Please see www.beneluxa.org for further information.

We have received a Patient Organisation Submission from Spinal Muscular Atrophy Ireland Foundation (SMA Ireland) about onasemnogene abeparvovec 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 it is not clear that onasemnogene abeparvovec 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 onasemnogene abeparvovec. The HSE makes the final decision on reimbursement.

Where can I get more information?

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

- the NCPE Technical Summary Document

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