



NCPE Plain English Summary

Drug name: atidarsagene autotemcel for the treatment of children with metachromatic leukodystrophy

Brand name: Libmeldy®

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 atidarsagene autotemcel used for?

Atidarsagene autotemcel is a medicine used to treat children with metachromatic leukodystrophy (MLD). MLD is a rare inherited disorder in which there is a change (mutation) in a gene needed to make an enzyme called arylsulfatase A (ARSA). Atidarsagene autotemcel is used in children with MLD who have mutations in the ARSA gene. It is given to

- those with late infantile or early juvenile forms of the disease who have not yet developed symptoms;
- those with early juvenile MLD who have initial symptoms but can still walk independently and have not yet developed mental deterioration.

What recommendation has the NCPE made to the HSE?

We have recommended that the HSE should consider not funding atidarsagene autotemcel 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 Rare Diseases Ireland about atidarsagene autotemcel 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 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. The HSE considers a number of factors along with our recommendation when deciding whether to provide this medicine. These factors are listed in the Health (Pricing and Supply of Medical Goods) Act 2013.

Next steps

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

Where can I get more information?

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

- the NCPE Technical Summary Document
- Libmeldy® European Public Assessment Report (EPAR) – [Summary for the public](#)
or
- searching for atidarsagene autotemcel on our website (www.ncpe.ie);
- searching for atidarsagene autotemcel 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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