

# NCPE Assessment

Plain English Summary

October, 2023

**Drug name:** Vosoritide  
(pronounced Vos-or-itide) for the  
treatment of achondroplasia.

**Brand name:** Voxzogo<sup>®</sup>

**HTA ID:** 22028

## What is the NCPE?

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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?

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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 Vosoritide used for?

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Achondroplasia is the most common form of disproportionate short stature. Although achondroplasia may be inherited approximately 80% of cases occur sporadically. Vosoritide may increase growth and is indicated for the treatment of achondroplasia in patients two years and older.

## What recommendation has the NCPE made to the HSE?

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We have recommended that the HSE should consider not funding vosoritide. 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 Little People of Ireland about vosoritide and shared it with the HSE. This submission will form part of the data that the HSE considers.

## Why did we make this recommendation?

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After reviewing the data presented by the pharmaceutical company, we concluded that there was insufficient clinical and economic evidence to support a reimbursement recommendation.

## Next steps

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

## Where can I get more information?

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

- the NCPE Technical Summary Document
- Voxzogo European Public Assessment Report (EPAR) –[Summary for the public](#), or
- searching for vosoritide on our website ([www.ncpe.ie](http://www.ncpe.ie));
- searching for vosoritide on the European Medicines Agency (EMA) website ([www.ema.europa.eu](http://www.ema.europa.eu)).

Please refer to the NCPE website for updated information on the reimbursement status of this medicine