Original French opinion adopted by the Transparency Committee

The legally binding text is the original French opinion version.

SUMMARY

Vosoritide

VOXZOGO 0.4 mg, 0.56 mg and 1.2 mg

powder and solvent for solution for injection, subcutaneous

Modification of listing conditions
Reassessment at the request of the TC

Adopted by the Transparency Committee on 12 February 2025

Summary of opinion

Favourable opinion for reimbursement in "the treatment of achondroplasia in patients from *4 months to under 2 years of age* whose epiphyses are not closed."

Favourable opinion for maintenance of reimbursement in "the treatment of achondroplasia in patients *2 years to under 5 years of age* whose epiphyses are not closed."

The population 5 years of age and older is not concerned by this new assessment.

Transparency Committee's Conclusions

Clinical Benefit

Indication extension in patients from 4 months to under 2 years of age and reassessment in patients from 2 years to under 5 years of age

- Achondroplasia is a rare and serious condition, with multiple, very frequent complications (functional, respiratory, neurological, psychological, etc.).
- This is a curative medicinal product.
- In view of the quality of the evidence concerning its efficacy, the Committee considers that the efficacy/adverse effects ratio of vosoritide has not been adequately established (see discussion):
  - For patients from 4 months to under 2 years of age (indication extension);
  - For patients from 2 years to under 5 years of age (the new data supplied in the context of the reassessment are of a nature to modify the Committee's previous conclusions, which were based on highly preliminary data).

  In fact, although the final results of controlled trial 111-206 having included patients under 5 years of age suggest a positive effect of vosoritide after 1 year for certain height measurement

endpoints, it is not possible to reach a robust conclusion with respect to its superiority versus placebo given inconclusive results for the primary endpoint, which was the variation in height Z-score (no statistically significant difference), in the three age cohorts.

➔ This is a first-line treatment in the absence of available therapies.

➔ **Public health benefit**

Considering:

- the seriousness of the disease and its low prevalence,
- the identified unmet medical need,
- the partial response to the identified need given:
  - the lack of robust evidence of an additional impact on morbidity and mortality and on quality of life,
  - the lack of data supporting an absence of deterioration in the care or life pathway, in the absence of robust quality of life data, and given the administration methods and adverse effects inherent to this in children,

VOXZOGO (vosoritide) is unlikely to have an additional impact on public health.

Considering all of these elements, the Committee deems that the clinical benefit of VOXZOGO (vosoritide) powder and solvent for solution for subcutaneous injection is low within the scope of the MA indication covered by this assessment:

- **Patients from 4 months to under 2 years of age (MA indication extension);**
- **Patients from 2 years to under 5 years of age (the new data supplied in the context of the reassessment are of a nature to modify the Committee's previous conclusions).**

The Committee issues a favourable opinion:

- **For inclusion of VOXZOGO (vosoritide) powder and solvent for solution for subcutaneous injection in both the list of covered drugs for outpatients and the list of covered drugs for inpatients approved for use in the MA indication extension in patients from 4 months to under 2 years of age and at the MA dosages.**
- **For maintenance of inclusion of VOXZOGO (vosoritide) powder and solvent for solution for subcutaneous injection in both the list of covered drugs for outpatients and the list of covered drugs for inpatients approved for use in the MA indication in patients from 2 years to under 5 years of age.**

➔ **Recommended reimbursement rate for inclusion in the list of covered drugs for outpatients: 65%**

Clinical Added Value

Considering:

- the results of a randomised, double-blind, placebo-controlled phase 2 study having evaluated vosoritide in patients from 4 months to under 5 year of age, which do not enable a robust conclusion to be reached concerning any superiority compared to placebo after 52 weeks of treatment for the main growth endpoints assessed, in particular the clinically relevant primary endpoint, which was variation in height Z-score (difference not statistically significant), both in the cohort of patients from 4 months to under 2 years of age and in the cohort of patients from 2 years to under 5 years of age,

- the level of evidence of these new data in patients from 2 years to under 5 years, which is superior to that of the data available for the initial assessment, which were purely descriptive and concerned 4 patients,
- the absence of a demonstrated benefit on quality of life in this population, despite this being particularly impacted in this condition,
- the major methodological limitations of the external comparisons supplied, which do not enable any formal conclusion to be reached with respect to a potential longer-term benefit of vosoritide versus no treatment,
- the safety profile, globally similar to that already observed in patients 5 years of age and older, but marked by more injection site reactions, particularly in the youngest patients,

the Committee deems that VOXZOGO (vosoritide) powder and solvent for solution for subcutaneous injection provides no clinical added value (CAV V) compared with the usual management of achondroplasia within the scope of the MA indication covered by this assessment:

- **Patients from 4 months to under 2 years of age (indication extension);**
- **Patients from 2 years to under 5 years of age (the new data supplied in the context of the reassessment are of a nature to modify the Committee's previous conclusions).**

© Haute Autorité de santé – **February 2025**