HAUTE AUTORITÉ DE SANTÉ

TRANSPARENCY COMMITTEE
SUMMARY
15 DECEMBER 2021

The legally binding text is the original French opinion version

vosoritide
VOXZOGO 0.4 mg, 0.56 mg and 1.2 mg solution for injection

First assessment

Key points

Favourable opinion for reimbursement in the treatment of achondroplasia in patients 2 years of age and older whose epiphyses are not closed. The diagnosis of achondroplasia should be confirmed by appropriate genetic testing.

What therapeutic improvement?

Therapeutic improvement in the current management of patients with achondroplasia.

Role in the care pathway?

Role of the medicinal product in the care pathway

The proprietary medicinal product VOXZOGO (vosoritide), as a daily subcutaneous injection, is the first treatment with an MA in achondroplasia.

It is a first-line treatment in patients 2 years of age and older with achondroplasia whose epiphyses are not closed. It should be initiated as early as possible. According to the SPC, this medicinal product should be discontinued once the patient has stopped growing.

VOXZOGO (vosoritide) should be initiated and directed by a physician appropriately qualified in the management of growth disorders or skeletal dysplasias.

COMMITTEE'S CONCLUSIONS

Considering all of this information and further to debate and voting, the Committee considers:

Clinical benefit

◗ Achondroplasia is a rare and serious condition, in which the complications are multiple and very common (functional, respiratory, neurological, psychological complications, etc.).
◗ The medicinal product VOXZOGO (vosoritide) is a curative medicinal product.
◗ The efficacy/adverse effects ratio of vosoritide is high.
◗ There are currently no therapeutic alternatives in the treatment of achondroplasia (see section 05).
◗ VOXZOGO (vosoritide) is a first-line treatment.

Public health impact

Considering:

- the seriousness of the condition,
- its rarity, with a prevalence estimated to be between 3.72 and 4.6/100,000 births in Europe,
- the unmet medical need,
- the partial response to the identified need given:
    - a demonstrated additional impact on morbidity in terms of height increase, but without robust data on limb disproportionality and the other clinical manifestations of achondroplasia,
    - the absence of an additional impact in terms of 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 these elements, the Committee deems that the clinical benefit of VOXZOGO (vosoritide) is substantial in the MA indication.

The Committee issues a favourable opinion for inclusion in both the hospital formulary list and the retail formulary list of reimbursed proprietary medicinal products approved for use in the marketing authorisation indication and dosages.

◗ **Recommended reimbursement rate: 65%**

Clinical Added Value

Considering:

- demonstration of the superiority of vosoritide compared to placebo in terms of annual height increase, a clinically relevant primary endpoint, with an effect size that is also clinically relevant (+1.57 cm/year) in a randomised double-blind study in children over 5 years of age,
- the unmet medical need in this rare and disabling genetic condition, for which management to date has been limited to symptomatic treatment,

And despite:

- data available in only four children between the ages of 2 and 5 years, although there is a benefit in treating this condition as early as possible,
- the absence of robust data relative to quality of life, which is particularly impacted in this condition,
- uncertainties with respect to the long-term safety profile of VOXZOGO (vosoritide) and the maintenance of its efficacy beyond the age of 2 years, in the absence of data,
- the absence of available data on the impact of this treatment on the other clinical manifestations of the condition,

The Committee considers that VOXZOGO (vosoritide) provides a moderate clinical added value (CAV III), in the care pathway compared to the current management, in the treatment of achondroplasia in patients 2 years of age and older whose epiphyses are not closed.