24 June 2021
EMA/CHMP/337860/2021
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Voxzogo

vosoritide

On 24 June 2021, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Voxzogo[2], intended for the treatment of achondroplasia in patients aged 2 years of age and above whose epiphyses are not closed.

The applicant for this medicinal product is BioMarin International Limited.

Voxzogo will be available as 0.4 mg, 0.56 mg and 1.2 mg vials of powder and a solvent for solution for injection. The active substance of Voxzogo is vosoritide, a modified type C natriuretic peptide (CNP) (ATC code: M05BX07). Like CNP, vosoritide acts as a positive regulator of endochondral bone growth by promoting chondrocyte proliferation and differentiation.

The benefits of Voxzogo are its ability to provide significant improvements in growth, adding an average of 1.57 cm in height a year in patients treated with Voxzogo 15 µg/kg/day. The observed increase in growth occurred proportionally in both the spine and the lower limbs. The most common side effects are hypotension, injection site reactions and vomiting.

The full indication is:

> Voxzogo is indicated for 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.

Voxzogo should be prescribed by physicians experienced in the treatment of growth disorders or skeletal dysplasias.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained

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