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Voxzogo (*vosoritide*)

An overview of Voxzogo and why it is authorised in the EU

What is Voxzogo and what is it used for?

Voxzogo is a medicine for treating achondroplasia in patients aged 4 months and older whose bones are still growing.

Achondroplasia is an inherited disease caused by a mutation (change) in a gene called fibroblast growth-factor receptor 3 (*FGFR3*). The mutation affects growth of almost all bones in the body including the skull, spine, arms and legs, resulting in very short stature with a characteristic appearance.

Achondroplasia is rare, and Voxzogo was designated an 'orphan medicine' (a medicine used in rare diseases) on 24 January 2013. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/EU3121094.

Voxzogo contains the active substance vosoritide.

How is Voxzogo used?

The medicine can only be obtained with a prescription. Treatment with Voxzogo must be started and supervised by a doctor experienced in the treatment of growth disorders or abnormal growth of bones.

Voxzogo is given as an injection under the skin once a day, preferably around the same time of day. The site of injection should be changed with each injection. The recommended dose is calculated according to the patient's body weight.

Treatment should only be started when achondroplasia is confirmed by genetic testing and should end when the patient is not likely to grow any further.

Voxzogo injections can be given by the patients' caregiver once they have been trained appropriately.

For more information about using Voxzogo, see the package leaflet or contact your doctor or pharmacist.

How does Voxzogo work?

In patients with achondroplasia, the *FGFR3* gene, which regulates growth, is permanently 'switched on'. This prevents normal growth of bones, leading to bones that are shorter than normal. The active

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substance in Voxzogo, vosoritide, works by binding to a receptor (target) called natriuretic peptide receptor type B (NPR-B), which reduces the activity of *FGFR3*. This stimulates growth of bones, thereby improving the symptoms of the disease.

What benefits of Voxzogo have been shown in studies?

Voxzogo was more effective than placebo (dummy treatment) at increasing growth rate after 52 weeks of treatment in a study involving 121 children aged from 5 to 17 years with confirmed achondroplasia. Children who received Voxzogo grew about 1.57 cm more during the year of treatment than those who received placebo. In addition, the results suggest that the improvement in growth is maintained.

Voxzogo was more effective than placebo at increasing growth rate in a second study involving 75 children aged from 4 months to less than 5 years with confirmed achondroplasia. The main measure of effectiveness was change in height Z-score, a measure which compares the patient's height to the average expected for their age and gender. After one year of treatment, children given Voxzogo had on average a greater improvement in height Z-score than those given placebo. Children who received Voxzogo also grew about 0.8 cm more during the year of treatment than those who received placebo.

What are the risks associated with Voxzogo?

For the full list of side effects and restrictions with Voxzogo, see the package leaflet.

The most common side effects with Voxzogo (which may affect more than 1 in 10 people) are injection site reactions (such as swelling, redness, itching or pain), vomiting and decreased blood pressure.

Why is Voxzogo authorised in the EU?

Voxzogo is effective at increasing the growth rate in children with achondroplasia aged 4 months and older. This may increase final height, allowing persons with achondroplasia to perform daily activities more easily. The side effects of the medicine are considered manageable. The safety profile in children below 5 years of age is similar to that in older children. The European Medicines Agency decided that Voxzogo's benefits are greater than its risks and it can be authorised for use in the EU.

What measures are being taken to ensure the safe and effective use of Voxzogo?

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Voxzogo have been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Voxzogo are continuously monitored. Suspected side effects reported with Voxzogo are carefully evaluated and any necessary action taken to protect patients.

Other information about Voxzogo

Voxzogo received a marketing authorisation valid throughout the EU on 26 August 2021.

Further information on Voxzogo can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/voxzogo

This overview was last updated in 10-2023.