BlueCross BlueShield
Federal Employee Program

VOXZOGO
(vosoritide)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Voxzogo (vosoritide) is indicated for the treatment of achondroplasia. Achondroplasia is the most common form of dwarfism in humans. Fibroblast growth factor receptor 3 (FGFR3) is a gene expressed in chondrocytes (cells found in cartilage) and mature osteoblasts. FGFR3 functions to regulate skeletal growth. Increased activation of FGFR3 after birth inhibits chondrocyte proliferation and disproportionately affects growth of long bones. Achondroplasia is usually identified at birth due to the presentation of shortened long bones, typically expressed in the arms and legs. Voxzogo is a peptide medication that inhibits the downstream effects caused by over-expression of FGFR3 and thereby improving endochondral bone growth (1).

Regulatory Status

FDA-approved indication: Voxzogo is a C type natriuretic peptide (CNP) analog indicated to increase linear growth in pediatric patients with achondroplasia with open epiphyses (1).

In clinical studies, Voxzogo was associated with transient decreases in blood pressure. Patients receiving Voxzogo were instructed to have adequate food intake and to be well-hydrated before administration to reduce the risk of a decrease in blood pressure and associated symptoms (1).

One of the inactive ingredients in Voxzogo is polysorbate 80. Thus, any patient with a hypersensitivity to polysorbate 80 should not use Voxzogo (1).

The Clinical Practice Guidelines for Achondroplasia include diagnostic criteria for achondroplasia, including genetic testing, x-ray findings, and clinical symptoms (2).

Summary

Voxzogo (vosoritide) is indicated for the treatment of achondroplasia. Achondroplasia is a genetic disease resulting from mutations in the fibroblast growth factor receptor 3 (FGFR3) gene. The mutation profoundly suppresses elongation of the long bones and thus patients with the condition typically present with inappropriately shortened arms and legs. Voxzogo inhibits the downstream effects of FGFR3 thereby promoting bone elongation and promotes normal stature. Patients receiving infusion were at risk of transient decreases in blood pressure and were advised to

consume adequate food and 240 to 300 mL of fluid in the hour prior to administration (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Voxzogo while maintaining optimal therapeutic outcomes.

References

1. Voxzogo [package insert]. Novato, CA: BioMarin Pharmaceutical, Inc.; November 2024.
2. Kubota T, Adachi M, et al. Clinical Practice Guidelines for Achondroplasia. *Clin Pediatr Endocrinol*. 2020;29(1):25-42.