Pharmacy Policy Bulletin: J-1178 Voxzogo (vosoritide) – Commercial and Healthcare Reform	
Number: J-1178	**Category:** Prior Authorization
Line(s) of Business: ☒ Commercial ☒ Healthcare Reform ☐ Medicare	**Benefit(s):** **Commercial:** **Prior Authorization (1.):** **1.** Miscellaneous Specialty Drugs Injectable = Yes w/ Prior Authorization **Healthcare Reform:** Not Applicable
Region(s): ☒ All ☐ Delaware ☐ New York ☐ Pennsylvania ☐ West Virginia	**Additional Restriction(s):** None
Version: J-0483-005	**Original Date:** 01/26/2022
Effective Date: 02/14/2025	**Review Date:** 01/29/2025

Drugs Product(s):	• Voxzogo (vosoritide)
FDA-Approved Indication(s):	• To increase linear growth in pediatric patients with achondroplasia with open epiphyses

Background:	• Voxzogo (vosoritide) is a self-administered, subcutaneous C type natriuretic peptide (CNP) analog. • Achondroplasia is the most commonly occurring abnormality of bone dysplasia in humans, occurring in 1 in 20,000 to 30,000 live births. This autosomal dominant genetic disorder is caused by a mutation in the fibroblast growth factor receptor 3 (*FGFR3*) gene. About 80% of cases are the result of new pathogenic variants, while the remaining are inherited. Achondroplasia results in disproportionate short stature and disordered architecture in the long bones, spine, face, and base of the skull. Complications associated with achondroplasia include leg bowing, narrowing of the lumbar spine, obesity, sleep-disordered breathing, recurrent otitis media, and cervical medullary compression. Achondroplasia affects males and females in equal numbers. If the bones that join the head and neck do not compress the brainstem or upper spinal cord, life expectancy is near normal. Voxzogo offers a treatment option that targets the underlying cause of the short stature. • In patients with achondroplasia endochondral bone growth, an essential process by which bone tissue is created, is negatively regulated due to a gain of function mutation in the *FGFR3* gene. Voxzogo, a CNP analog, acts as a positive regulator of the signaling pathway downstream of *FGFR3* to promote endochondral bone growth. • Before the availability of Voxzogo, the management of achondroplasia focused on maximizing functional capacity and monitoring, preventing, and treating complications. There were no pharmacologic interventions available. However, at present it is unknown if Voxzogo can prevent hearing loss, sleep apnea, and life-threatening skeletal problems that result from achondroplasia.

	• Prescribing Considerations: ○ Recommended dosage is based on patient's weight; monitor growth and adjust dosage according to body weight. ○ Pediatric endocrinologist prescribing is recommended. ○ Use of Voxzogo for this indication is supported by evidence from a study in pediatric patients aged 5 to 15 years with achondroplasia, pharmacokinetic data in pediatric patients aged 4.5 months to 15 years, and additional safety data in pediatric patients aged 4.4 months to < 5 years. Permanently discontinue Voxzogo upon closure of the epiphyses. ○ Ensure adequate food and fluid intake prior to administration.

Approval Criteria

I. Initial Authorization

When a benefit, coverage of Voxzogo may be approved when all of the following criteria are met **(A. through D.)**:

A. The member is 17 years of age or younger.

B. The member has a diagnosis of achondroplasia (ICD-10: Q77.4), with documentation of genetic testing for the *FGFR3* gene mutation.

C. The prescriber provides a baseline annualized growth velocity (AGV).

D. The prescriber attests that the member's epiphyses have not closed.

II. Reauthorization

When a benefit, reauthorization of Voxzogo may be approved when all of the following criteria are met **(A. and B.)**:

A. The prescriber provides documentation of an increase in AGV compared to baseline.

B. The prescriber attests that the member's epiphyses have not closed.

III. An exception to some or all of the criteria above may be granted for select members and/or circumstances based on state and/or federal regulations.

Limitations of Coverage

I. Use of growth hormone is not recommended in patients with achondroplasia as it can potentially worsen the disproportion seen in these patients.

II. Coverage of drug(s) addressed in this policy for disease states outside of the FDA-approved indications should be denied based on the lack of clinical data to support effectiveness and safety in other conditions unless otherwise noted in the approval criteria.

III. For Commercial or HCR members with a closed formulary, a non-formulary product will only be approved if the member meets the criteria for a formulary exception in addition to the criteria outlined within this policy.

Authorization Duration

- Commercial and HCR Plans: If approved, up to a 12 month authorization may be granted.

Automatic Approval Criteria

None.

References:

1. Voxzogo [package insert]. Novato: CA: BioMarin Pharmaceutical Inc.; November 2024.
2. UpToDate. Achondroplasia. Available at: https://www.uptodate.com. Accessed January 4, 2024.