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Section:	Prescription Drugs	**Effective Date:**	April 1, 2025
Subsection:	Endocrine and Metabolic Drugs	**Original Policy Date:**	December 10, 2021
Subject:	Voxzogo	**Page:**	1 of 5

Last Review Date: March 7, 2025

Voxzogo

Description

Voxzogo (vosoritide)

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).

Related policies

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Voxzogo may be considered **medically necessary** if the conditions indicated below are met.

Voxzogo may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 17 years of age or under
18 years of age or older **WITH** open epiphyses

Diagnosis

Patient must have the following:

1. Achondroplasia
    a. Diagnosis has been confirmed by **ONE** of the following:
        i. Genetic testing for FGFR3 mutation
        ii. X-ray findings and clinical symptoms (e.g., short stature with marked shortening of extremities) consistent with achondroplasia

AND ALL of the following:

a. Patient has open epiphyses, confirmed by diagnostic imaging
b. Prescribed by or recommended by a prescriber with experience treating achondroplasia (e.g., endocrinologist)
c. Prescriber agrees to monitor body weight, growth, and physical development
d. Prescriber agrees to advise patient and/or patient's caregivers to have adequate food intake and 240-300 mL of fluid in the hour prior to administration

e. Prescriber agrees to discontinue Voxzogo upon confirmation of no further growth potential (i.e., closure of epiphyses)
f. **NOT** used in combination with a growth hormone agent

Prior–Approval *Renewal* Requirements

Age 17 years of age or under
18 years of age or older **WITH** open epiphyses

Diagnosis

Patient must have the following:

1. Achondroplasia
    a. Patient has had clinical benefit while on therapy (e.g., increased linear growth)

AND ALL of the following:
a. Patient has open epiphyses, confirmed by diagnostic imaging
b. Prescribed by or recommended by a prescriber with experience treating achondroplasia (e.g., endocrinologist)
c. Prescriber agrees to monitor body weight, growth, and physical development
d. Prescriber agrees to advise patient and/or patient's caregivers to have adequate food intake and 240-300 mL of fluid in the hour prior to administration
e. Prescriber agrees to discontinue Voxzogo upon confirmation of no further growth potential (i.e., closure of epiphyses)
f. **NOT** used in combination with a growth hormone agent

Policy Guidelines

Pre–PA Allowance

None

Prior–Approval Limits

Quantity

Voxzogo strength	Quantity
0.4 mg	90 vials per 90 days
0.56 mg	
1.2 mg	

Duration 12 months

Prior – Approval *Renewal* Limits

Same as above

Rationale

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.

Policy History

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Date	Action
December 2021	Addition to PA
March 2022	Annual review and reference update. Per SME: Added initiation requirement for the diagnosis to be confirmed by genetic testing for FGFR3 mutation or by x-ray findings and clinical symptoms; Added renewal requirement of patient has had clinical benefit from therapy; Changed durations from 6 months to 12 months; Added that open epiphyses must be confirmed by diagnostic imaging; Added requirement of “prescribed by or recommended by a prescriber with experience treating achondroplasia (e.g., endocrinologist);” Added requirement of “not used in combination with a growth hormone agent;” Added statement to regulatory status regarding polysorbate hypersensitivity.
November 2023	Per PI update, revised age requirement to 17 years of age or under OR age 18 or older with open epiphyses. Changed policy number to 5.30.079
December 2023	Annual review
March 2024	Annual review
March 2025	Annual review and reference update

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 7, 2025 and is effective on April 1, 2025.