**Effective Date ..... 3/1/2025**
**Coverage Policy Number.....IP0402**

# Vosoritide

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## Related Coverage Resources

***INSTRUCTIONS FOR USE***

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## Overview

This policy supports medical necessity review for vosoritide (**Voxzogo**™) subcutaneous injection.

## Medical Necessity Criteria

**Vosoritide (Voxzogo) is considered medically necessary when the following are met:**

**Achondroplasia.** Individual meets **ALL** of the following:

A. Less than 18 years of age
B. Documented diagnosis of achondroplasia is confirmed by genetic testing with an identifiable pathogenic variant in the fibroblast growth factor receptor type 3 (*FGFR3*) gene
C. Epiphyses are open

D. Individual will not have limb-lengthening surgery during treatment with vosoritide (Voxzogo)
E. The medication is prescribed by or in consultation with an endocrinologist or medical geneticist

When coverage is available and medically necessary, the dosage, frequency, duration of therapy, and site of care should be reasonable, clinically appropriate, and supported by evidence-based literature and adjusted based upon severity, alternative available treatments, and previous response to therapy.

Receipt of sample product does not satisfy any criteria requirements for coverage.

## Reauthorization Criteria

Continuation of vosoritide (Voxzogo) is considered medically necessary for Achondroplasia when the above medical necessity criteria are met AND there is documentation of beneficial response.

## Authorization Duration

Initial approval duration: up to 12 months
Reauthorization approval duration: up to 12 months

## Conditions Not Covered

Any other use is considered experimental, investigational, or unproven, including the following (this list may not be all inclusive):

1. **Hypochondroplasia, Thanatophoric Dysplasia, or other Short Stature Conditions other than Achondroplasia (e.g., trisomy 21, pseudoachondroplasia).**
   Voxzogo is only indicated for patients with achondroplasia.[1] There is no evidence Voxzogo is effective for other short stature conditions.

2. **Concurrent Treatment with Growth Hormone (e.g., somatropin), Long-Acting Growth Hormone (e.g., Ngenla® [somatrogon-ghla], Skytrofa® [lonapegsomatropin], Sogroya® [somapacitan-beco]), or Insulin-like Growth Factor- 1 (IGF-1) [i.e., Increlex® (mecasermin)] Agents.**
   Growth hormone agents and Increlex are NOT indicated to increase growth in patients with achondroplasia. [6-10 Additionally], there are no available studies demonstrating the safety or efficacy of concurrent use with Voxzogo.

## Background

**OVERVIEW**
Voxzogo, a C type natriuretic peptide (CNP) analog, is indicated to increase linear growth in pediatric patients with achondroplasia with open epiphyses.1

**Disease Overview**
Achondroplasia is the most common form of disproportionate short stature in humans.2 It is a primary skeletal dysplasia caused by a mutation in the fibroblast growth factor receptor 3 (FGFR3) gene; this mutation leads to impaired endochondral ossification. Achondroplasia occurs in approximately 1 in 20,000 to 30,000 live births.3 It occurs as a result of a spontaneous mutation in 80% of patients (i.e., both parents are of normal height).4 In the remaining 20% of patients, the mutation is inherited from a parent. Achondroplasia is characterized by short stature, long-

bone shortening in the proximal upper and lower extremities, and macrocephaly. The diagnosis can be confirmed by molecular testing.5 In the pivotal trial for Voxzogo, achondroplasia was confirmed by genetic testing in all patients.2 Additionally, exclusion criteria included evidence of decreased growth velocity (< 1.5 cm/year) or of growth plate closure through bilateral lower extremity X-rays.

## References


1. Voxzogo™ subcutaneous injection [prescribing information]. Novato, CA: BioMarin; November 2024.
2. Once-daily, subcutaneous vosoritide therapy in children with achondroplasia: a randomised, double-blind, phase 3, placebo-controlled, multicentre trial. Lancet. 2020;396(10252):684-692.
3. National Organization for Rare Disorders (NORD). Achondroplasia Last updated November 17, 2023. Available at: Achondroplasia - NORD (National Organization for Rare Disorders) (rarediseases.org). Accessed on November 18, 2024.
4. Achondroplasia: a comprehensive clinical disease. Orphanet J Rare Dis. 2019;14(1):1.
5. Health supervision for people with achondroplasia. American Academy of Pediatrics. Pediatrics. 2020;145(6): e20201010.
6. Norditropin® subcutaneous injection [prescribing information]. Plainsboro, NJ: Novo Nordisk; March 2020.
7. Skytrofa™ subcutaneous injection [prescribing information]. Princeton, NJ: Ascendis Pharma; May 2024.
8. Sogroya® subcutaneous injection [prescribing information]. Plainsboro, NJ: Novo Nordisk; April 2023.
9. Ngenla® subcutaneous injection [prescribing information]. New York, NY: Pfizer; June 2023.
10. Increlex® subcutaneous injection [prescribing information]. Cambridge, MA: Ipsen; March 2024.
11. Dauber A, Zhang A, Kanakatti Shankar R, et al. Vosoritide treatment for children with hypochondroplasia: a phase 2 trial. EClinicalMedicine. 2024; 71:102591.


| Type of Revision | Summary of Changes | Date |
|---|---|---|
| Annual Revision | No criteria changes. | 3/1/2025 |

The policy effective date is in force until updated or retired.