

Drug and Biologic Coverage Policy



Effective Date 3/1/2024
Next Review Date... 3/1/2025
Coverage Policy Number IP0402

Vosoritide

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Related Coverage Resources

INSTRUCTIONS FOR USE

The following Coverage Policy applies to health benefit plans administered by Cigna Companies. Certain Cigna Companies and/or lines of business only provide utilization review services to clients and do not make coverage determinations. References to standard benefit plan language and coverage determinations do not apply to those clients. Coverage Policies are intended to provide guidance in interpreting certain standard benefit plans administered by Cigna Companies. Please note, the terms of a customer's particular benefit plan document [Group Service Agreement, Evidence of Coverage, Certificate of Coverage, Summary Plan Description (SPD) or similar plan document] may differ significantly from the standard benefit plans upon which these Coverage Policies are based. For example, a customer's benefit plan document may contain a specific exclusion related to a topic addressed in a Coverage Policy. In the event of a conflict, a customer's benefit plan document always supersedes the information in the Coverage Policies. In the absence of a controlling federal or state coverage mandate, benefits are ultimately determined by the terms of the applicable benefit plan document. Coverage determinations in each specific instance require consideration of 1) the terms of the applicable benefit plan document in effect on the date of service; 2) any applicable laws/regulations; 3) any relevant collateral source materials including Coverage Policies and; 4) the specific facts of the particular situation. Coverage Policies relate exclusively to the administration of health benefit plans. Coverage Policies are not recommendations for treatment and should never be used as treatment guidelines. In certain markets, delegated vendor guidelines may be used to support medical necessity and other coverage determinations.

Overview

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

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

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.

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 the 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; October 2023.
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 December 8, 2021. Available at: Achondroplasia - NORD (National Organization for Rare Disorders) (rarediseases.org). Accessed on October 30, 2023.
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]. Palo Alto, CA: Ascendis; October 2022.
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; October 2023.

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