PRIOR AUTHORIZATION POLICY

POLICY: Growth Disorders – Voxzogo Prior Authorization Policy
- Voxzogo™ (vosoritide subcutaneous injection – BioMarin)

REVIEW DATE: 11/19/2025

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. EACH COVERAGE REQUEST SHOULD BE REVIEWED ON ITS OWN MERITS. MEDICAL DIRECTORS ARE EXPECTED TO EXERCISE CLINICAL JUDGMENT WHERE APPROPRIATE AND HAVE DISCRETION IN MAKING INDIVIDUAL COVERAGE DETERMINATIONS. WHERE COVERAGE FOR CARE OR SERVICES DOES NOT DEPEND ON SPECIFIC CIRCUMSTANCES, REIMBURSEMENT WILL ONLY BE PROVIDED IF A REQUESTED SERVICE(S) IS SUBMITTED IN ACCORDANCE WITH THE RELEVANT CRITERIA OUTLINED IN THE APPLICABLE COVERAGE POLICY, INCLUDING COVERED DIAGNOSIS AND/OR PROCEDURE CODE(S). REIMBURSEMENT IS NOT ALLOWED FOR SERVICES WHEN BILLED FOR CONDITIONS OR DIAGNOSES THAT ARE NOT COVERED UNDER THIS COVERAGE POLICY (SEE "CODING INFORMATION" BELOW). WHEN BILLING, PROVIDERS MUST USE THE MOST APPROPRIATE CODES AS OF THE EFFECTIVE DATE OF THE SUBMISSION. CLAIMS SUBMITTED FOR SERVICES THAT ARE NOT ACCOMPANIED BY COVERED CODE(S) UNDER THE APPLICABLE COVERAGE POLICY WILL BE DENIED AS NOT COVERED. 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.

CIGNA NATIONAL FORMULARY COVERAGE:

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.

POLICY STATEMENT

Prior Authorization is recommended for prescription benefit coverage of Voxzogo. All approvals are provided for the duration noted below. Because of the specialized skills required for evaluation and diagnosis of patients treated with Voxzogo as well as the monitoring required for adverse events and long-term efficacy, approval requires Voxzogo to be prescribed by or in consultation with a physician who specializes in the condition being treated.

- **Voxzogo™ (vosoritide subcutaneous injection - BioMarin)**

is(are) covered as medically necessary when the following criteria is(are) met for FDA-approved indication(s) or other uses with supportive evidence (if applicable):

FDA-Approved Indication

1. **Achondroplasia.** Approve for 1 year if the patient meets ONE of the following (A or B):
   - **A)** Initial Therapy or Patient Has Been on Voxzogo for < 1 Year. Approve if the patient meets ALL of the following (i, ii, iii, iv, v, and vi):
     - **i.** Patient is < 18 years of age; AND
     - **ii.** The diagnosis of achondroplasia has been confirmed by genetic testing with an identifiable mutation in the fibroblast growth factor receptor type 3 (FGFR3) gene; AND
     - **iii.** Patient's epiphyses are open; AND
     - **iv.** Patient will not have limb-lengthening surgery during treatment with Voxzogo; AND
     - **v.** The prescriber has confirmed the patient is able to drink approximately 240 to 300 mL of fluid in the hour prior to Voxzogo administration; AND
     - **vi.** The medication is prescribed by or in consultation with a pediatric endocrinologist; OR
   - **B)** Patient Has Been Receiving Voxzogo for ≥ 1 Year. Approve if the patient meets ALL of the following (i, ii, iii, iv, v, vi, and vii):
     - **i.** Patient is < 18 years of age; AND
     - **ii.** The diagnosis of achondroplasia has been confirmed by genetic testing with an identifiable mutation in the fibroblast growth factor receptor type 3 (FGFR3) gene; AND
     - **iii.** Patient's epiphyses are open; AND
     - **iv.** Patient will not have limb-lengthening surgery during treatment with Voxzogo; AND
     - **v.** The prescriber has confirmed the patient is able to drink approximately 240 to 300 mL of fluid in the hour prior to Voxzogo administration; AND

vi. The medication is prescribed by or in consultation with a pediatric endocrinologist; AND

vii. Patient's most recent annualized growth velocity continues to be above their baseline annualized growth velocity value (i.e., before the patient started on Voxzogo).

CONDITIONS NOT COVERED

- **Voxzogo™ (vosoritide subcutaneous injection - BioMarin) is(are) considered not medically necessary for ANY other use(s) including the following (this list may not be all inclusive; criteria will be updated as new published data are available):**

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 a small published Phase 2 trial showing some efficacy for children with hypochondroplasia.[11] 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.

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 12, 2025.
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; July 2025.
7. Skytrofa™ subcutaneous injection [prescribing information]. Princeton, NJ: Ascendis Pharma; September 2025.
8. Sogroya® subcutaneous injection [prescribing information]. Plainsboro, NJ: Novo Nordisk; July 2025.
9. Ngenla® subcutaneous injection [prescribing information]. New York, NY: Pfizer; July 2025.
10. Increlex® subcutaneous injection [prescribing information]. Cambridge, MA: Ipsen; July 2025.
11. Dauber A, Zhang A, Kanakatti Shankar R, et al. Vosoritide treatment for children with hypochondroplasia: a phase 2 trial. *EClinicalMedicine*. 2024;71:102591.

HISTORY

Type of Revision	Summary of Changes	Review Date
Early Annual Revision	**Achondroplasia (initial and continuation criteria)**, the following criteria were removed: 1) The lower age limit of ≥ 5 years of age; and 2) The criterion that there is evidence of an annualized growth velocity ≥ 1.5 cm/year. **For Conditions Not Covered** : Additional examples of long-acting growth hormone products were added to Concurrent Treatment with Growth Hormone, Long-Acting Growth Hormone, or Insulin-like Growth Factor-1 Agents.	11/01/2023
Annual Revision	No criteria changes.	11/20/2024
Annual Revision	No criteria changes.	11/19/2025