

## Clinical Policy: Vosoritide (Voxzogo)

Reference Number: PA.CP.PHAR.525

Effective Date: 01/2023

Last Review Date: 01/2026

### Description

Vosoritide (Voxzogo<sup>®</sup>) is an analog of C-type natriuretic peptide (CNP).

### FDA Approved Indication(s)

Voxzogo is indicated to increase linear growth in pediatric patients with achondroplasia with open epiphyses.

This indication is approved under accelerated approval based on an improvement in annualized growth velocity. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s).

### Policy/Criteria

*Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.*

It is the policy of PA Health & Wellness<sup>®</sup> that Voxzogo is **medically necessary** when the following criteria are met:

#### I. Initial Approval Criteria

##### A. Achondroplasia (must meet all):

1. Diagnosis of achondroplasia with genetic testing confirming a mutation in the fibroblast growth factor receptor 3 (FGFR3) gene;
2. Prescribed by or in consultation with a pediatric endocrinologist;
3. Age between 4.4 months (*see Appendix D*) and 18 years;
4. At the time of request, radiographic evidence indicates open epiphyses (growth plates);
5. Documentation of baseline annualized growth velocity, calculated based on standing height measured over the course of 6 months prior to request;
6. Documentation of member's current weight (in kg);
7. Voxzogo is not prescribed concurrently with any human growth hormone products (e.g., Genotropin<sup>®</sup>, Humatrope<sup>®</sup>, Norditropin<sup>®</sup>, Nutropin AQ<sup>®</sup>, Omnitrope<sup>®</sup>, Saizen<sup>®</sup>, Zomacton<sup>®</sup>);
8. Dose does not exceed both of the following:
  - a. Weight-based daily dosing (*see Section V. Dosage and Administration*).
  - b. 1 vial per day.

**Approval duration: 6 months**

##### B. Other diagnoses/indications

1. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

## II. Continued Therapy

### A. Achondroplasia (must meet all):

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.PHARM.01) applies;
2. Member is responding positively to therapy as evidenced by improvement in annualized growth velocity from baseline;
3. Radiographic evidence within the last four months indicates that the member continues to have open epiphyses (growth plates);
4. Documentation of member's current weight (in kg);
5. If request is for a dose increase, new dose does not exceed both of the following"
  - a. Weight-based daily (*see Section V. Dosage and Administration*).
  - b. 1 vial per day.

**Approval duration: 6 months**

### B. Other diagnoses/indications (must meet 1 or 2):

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.PHARM.01) applies.

**Approval duration: Duration of request or 6 months (whichever is less); or**

2. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

## III. Diagnoses/Indications for which coverage is NOT authorized:

- ### A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – PA.CP.PMN.53.

## IV. Appendices/General Information

### *Appendix A: Abbreviation/Acronym Key*

CNP: C-type natriuretic peptide

FDA: Food and Drug Administration

FGFR3: fibroblast growth factor  
receptor 3

### *Appendix B: Therapeutic Alternatives*

Not applicable

### *Appendix C: Contraindications/Boxed Warnings*

None reported

### *Appendix D: General Information*

- Use of Voxzogo in the pediatric population is supported by evidence from an adequate and well-controlled study in 121 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.
  - 4.4 months is equivalent to approximately 4 months and 12 days.

**V. Dosage and Administration**

| Indication     | Dosing Regimen  | Maximum Dose                  |
|----------------|---|-------------------------------|
| Achondroplasia | Dose is a once-daily SC injection based on actual body weight: <ul style="list-style-type: none"> <li>• 3 kg: 0.096 mg/day;</li> <li>• 4 kg: 0.12 mg/day;</li> <li>• 5 kg: 0.16 mg/day;</li> <li>• 6-7 kg: 0.2 mg/day</li> <li>• 8-11 kg: 0.24 mg/day;</li> <li>• 12-16 kg: 0.28 mg/day;</li> <li>• 17-21 kg: 0.32 mg/day;</li> <li>• 22-32 kg: 0.4 mg/day;</li> <li>• 33-43 kg: 0.5 mg/day;</li> <li>• 44-59 kg: 0.6 mg/day;</li> <li>• 60-89 kg: 0.7 mg/day;</li> <li>• <math>\geq</math> 90 kg: 0.8 mg/day.</li> </ul> | Varies per actual body weight |

**VI. Product Availability**

Lyophilized powder in single-dose vials: 0.4 mg, 0.56 mg, 1.2 mg

**VII. References**

1. Voxzogo Prescribing Information. Novato, CA: BioMarin Pharmaceutical Inc.; November 2024. Available at: <https://hcp.biomin.com/>. Accessed October 21, 2025.
2. Savarirayan R, Irving M, Bacino CA, et al. C-type natriuretic peptide analogue in children with achondroplasia. *N Engl J Med*. 2019. 381(1):25-35. doi:10.1056/NEJMoa1813445.
3. Savarirayan R, Tofts L, Irving M, et al. Once-daily, subcutaneous vosoritide therapy in children with achondroplasia: a randomized, double-blind, phase 3, placebo-controlled, multicenter trial. *Lancet*. 2020; 396:684-92.
4. Hoover-Fong J, Scott CI, Jones MC, AAP Committee on Genetics. Health supervision for people with achondroplasia. *Pediatrics*. 2020;145(6):e20201010.
5. Savarirayan R, Ireland P, Irving M, et al. International Consensus Statement on the diagnosis, multidisciplinary management and lifelong care of individuals with achondroplasia. *Nat Rev Endocrinol*. 2022;18(3):173-189.

**Coding Implications**

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

| HCPCS Codes | Description                       |
|-------------|-----------------------------------|
| J3490       | Unclassified drugs                |
| C9399       | Unclassified drugs or biologicals |

| Reviews, Revisions, and Approvals  | Date    |
|--|---------|
| Policy created   | 01/2023 |
| 1Q 2024 annual review: RT4: updated criteria with pediatric age extension; added appendix D general information on use in pediatric population; references reviewed and updated. | 01/2024 |
| 1Q 2025 annual review: added HCPCS code for unclassified drugs or biologicals [J3490, C9399]; references reviewed and updated.   | 01/2025 |
| 1Q 2026 annual review: no significant changes; references reviewed and updated.  | 01/2026 |