Clinical Policy: Vosoritide (Voxzogo)

Reference Number: PA.CP.PHAR.525
Effective Date: 01/2023
Last Review Date: 01/2023

Revision Log

Description
Vosoritide (Voxzogo™) 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 who are 5 years of age and older 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® 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 5 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®, Humatrope®, Norditropin®, Nutropin AQ®, Omnitrope®, Saizen®, Zomacton®);
8. Dose does not exceed 1 vial per day and any of the following, based on actual body weight (a-h):
   a. 10-11 kg: 0.24 mg per day;
   b. 12-16 kg: 0.28 mg per day;
   c. 17-21 kg: 0.32 mg per day;
   d. 22-32 kg: 0.4 mg per day;
   e. 33-43 kg: 0.5 mg per day;
   f. 44-59 kg: 0.6 mg per day;
   g. 60-89 kg: 0.7 mg per day;

h. ≥ 90 kg: 0.8 mg 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.LTSS.PHAR.01) applies;
1. Member is responding positively to therapy as evidenced by improvement in annualized growth velocity from baseline;
2. Radiographic evidence within the last four months indicates that the member continues to have open epiphyses (growth plates);
3. Documentation of member's current weight (in kg);
4. If request is for a dose increase, new dose does not exceed 1 vial per day and any of the following, based on actual body weight (a-h):
   a. 10-11 kg: 0.24 mg per day;
   b. 12-16 kg: 0.28 mg per day;
   c. 17-21 kg: 0.32 mg per day;
   d. 22-32 kg: 0.4 mg per day;
   e. 33-43 kg: 0.5 mg per day;
   f. 44-59 kg: 0.6 mg per day;
   g. 60-89 kg: 0.7 mg per day;
   h. ≥ 90 kg: 0.8 mg 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.LTSS.PHAR.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

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
Achondroplasia	Dose is a once-daily SC injection based on actual body weight: • 10-11 kg: 0.24 mg/day; • 12-16 kg: 0.28 mg/day; • 17-21 kg: 0.32 mg/day; • 22-32 kg: 0.4 mg/day; • 33-43 kg: 0.5 mg/day; • 44-59 kg: 0.6 mg/day; • 60-89 kg: 0.7 mg/day; • ≥ 90 kg: 0.8 mg/day.	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 2021. Available at: www.voxzogo.com. Accessed November 2, 2022.
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.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created	01/2023	