Clinical Policy: Vosoritide (Voxzogo)

Reference Number: ERX.SPA.428
Effective Date: 11.19.21
Last Review Date: 02.22
Line of Business: Commercial, Medicaid

Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

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.

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.

Health plan approved formularies should be reviewed for all coverage determinations. Requirements to use preferred alternative agents apply only when such requirements align with the health plan approved formulary.

It is the policy of health plans affiliated with Envolve Pharmacy Solutions™ 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 any of the following, based on actual body weight (a-h) (1 vial per day):
   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 ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

II. Continued Therapy

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

1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions or member has previously met initial approval criteria;
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 any of the following, based on actual body weight (a-h) (1 vial per day):
   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 a health plan affiliated with Envolve Pharmacy Solutions and documentation supports positive response to therapy.
   Approval duration: Duration of request or 6 months (whichever is less);
2. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

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 policy – ERX.PA.01 or evidence of coverage documents.

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;	Varies per actual body weight

Indication	Dosing Regimen	Maximum Dose
	• 44-59 kg: 0.6 mg/day; • 60-89 kg: 0.7 mg/day; • ≥ 90 kg: 0.8 mg/day.	

VI. Product Availability

Lyophilized powder in single-dose vials for reconstitution: 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 19, 2021.
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.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created pre-emptively	01.05.21	02.21
1Q 2022 annual review: drug is now FDA approved – criteria updated per FDA labeling: applied the requirement for documentation of continued open epiphyses for reauthorization to all ages (not just for adults); added an exclusion for concomitant use with human growth hormone products; added a requirement for documentation of member's weight for dose calculation purposes; changed reauth duration from 12 months to 6 months; references reviewed and updated.	11.30.21	02.22

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information.

This Clinical Policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members.

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