Clinical Policy: Vosoritide (Voxzogo)

Reference Number: CP.PHAR.525
Effective Date: 11.19.21
Last Review Date: 02.24
Line of Business: Commercial, HIM, 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 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 health plans affiliated with Centene Corporation® 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®, Humatrope®, Norditropin®, Nutropin AQ®, Omnitrope®, Saizen®, Zomacton®);
8. Dose does not exceed 1 vial per day and weight-based daily dosing (*see Section V. Dosage and Administration*).

Approval duration: 6 months

B. Other diagnoses/indications (must meet 1 or 2)**:**
1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
   a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or
   b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

II. Continued Therapy

A. Achondroplasia (must meet all)**:**
1. Member meets one of the following (a or b):
   a. Currently receiving medication via Centene benefit or member has previously met initial approval criteria;
   b. Member is currently receiving medication and is enrolled in a state and product with continuity of care regulations (*refer to state specific addendums for CC.PHARM.03A and CC.PHARM.03B);*
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 1 vial per day and weight-based daily (*see Section V. Dosage and Administration*).

Approval duration: 6 months

B. Other diagnoses/indications (must meet 1 or 2)**:**
1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
   a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or
   b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business:

CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or

2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

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 – CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid, 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

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: • 3 kg: 0.096 mg/day; • 4 kg: 0.12 mg/day; • 5 kg: 0.16 mg/day; • 6-7 kg: 0.2 mg/day • 8-11 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: 0.4 mg, 0.56 mg, 1.2 mg

VII. References

1. Voxzogo Prescribing Information. Novato, CA: BioMarin Pharmaceutical Inc.; October 2023. Available at: www.voxzogo.com. Accessed November 1, 2023.
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 pre-emptively	01.05.21	02.21
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
Template changes applied to other diagnoses/indications.	10.04.22	
1Q 2023 annual review: no significant changes; references reviewed and updated. Template changes applied to continued therapy section.	11.02.22	02.23
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.	11.01.23	02.24

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. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. "Health Plan" means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan's affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment or medical care. It is not intended to dictate to providers how to practice medicine. 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. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.

This clinical policy is the property of the Health Plan. Unauthorized copying, use, and distribution of this clinical policy or any information contained herein are strictly prohibited. Providers, members and their representatives are bound to the terms and conditions expressed herein through the terms of their contracts. Where no such contract exists, providers, members and their representatives agree to be bound by such terms and conditions by providing services to members and/or submitting claims for payment for such services.

Note:
For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.