



An Independent Licensee of the Blue Cross Blue Shield Association

PHARMACY COVERAGE GUIDELINES  
SECTION: DRUGS

ORIGINAL EFFECTIVE DATE: 2/17/2022  
LAST REVIEW DATE:  
LAST CRITERIA REVISION DATE:  
ARCHIVE DATE:

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## VOXZOGO™ (vosoritide) injection

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Coverage for services, procedures, medical devices and drugs are dependent upon benefit eligibility as outlined in the member's specific benefit plan. This Pharmacy Coverage Guideline must be read in its entirety to determine coverage eligibility, if any.

This Pharmacy Coverage Guideline provides information related to coverage determinations only and does not imply that a service or treatment is clinically appropriate or inappropriate. The provider and the member are responsible for all decisions regarding the appropriateness of care. Providers should provide BCBSAZ complete medical rationale when requesting any exceptions to these guidelines.

The section identified as "**Description**" defines or describes a service, procedure, medical device or drug and is in no way intended as a statement of medical necessity and/or coverage.

The section identified as "**Criteria**" defines criteria to determine whether a service, procedure, medical device or drug is considered medically necessary or experimental or investigational.

State or federal mandates, e.g., FEP program, may dictate that any drug, device or biological product approved by the U.S. Food and Drug Administration (FDA) may not be considered experimental or investigational and thus the drug, device or biological product may be assessed only on the basis of medical necessity.

Pharmacy Coverage Guidelines are subject to change as new information becomes available.

For purposes of this Pharmacy Coverage Guideline, the terms "experimental" and "investigational" are considered to be interchangeable.

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This Pharmacy Coverage Guideline does not apply to FEP or other states' Blues Plans.

Information about medications that require precertification is available at [www.azblue.com/pharmacy](http://www.azblue.com/pharmacy).

Some large (100+) benefit plan groups may customize certain benefits, including adding or deleting precertification requirements.

All applicable benefit plan provisions apply, e.g., waiting periods, limitations, exclusions, waivers and benefit maximums.

Precertification for medication(s) or product(s) indicated in this guideline requires completion of the [request form](#) in its entirety with the chart notes as documentation. **All requested data must be provided.** Once completed the form must be signed by the prescribing provider and faxed back to BCBSAZ Pharmacy Management at (602) 864-3126 or emailed to [Pharmacyprecert@azblue.com](mailto:Pharmacyprecert@azblue.com). **Incomplete forms or forms without the chart notes will be returned.**



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### Criteria:

- **Criteria for initial therapy:** Voxzogo (vosoritide) is considered *medically necessary* and will be approved when **ALL** of the following criteria are met:
1. Prescriber is a physician specializing in the patient's diagnosis or is in consultation with an Endocrinologist or Pediatric Endocrinologist
  2. Individual is 5 years of age or older
  3. A confirmed diagnosis of achondroplasia in an individual who can stand and ambulate without assistance
  4. **ALL** of the following **baseline tests** have been completed before initiation of treatment with continued monitoring as clinically appropriate:
    - a. Pathogenic gain of function variant in fibroblastic growth factor receptor 3 (*FGFR3*) gene
    - b. Recent (within the last 12 months) radiographic evidence epiphyses have not closed
  5. Individual does not have hypochondroplasia or short stature condition other than achondroplasia
  6. Individual is not planning to have limb-lengthening surgery
  7. Individual does not have systolic blood pressure (BP) < 70 millimeters of mercury (mm Hg) or recurrent symptomatic hypotension (defined as episodes of low BP generally accompanied by symptoms such as, dizziness, fainting) or recurrent symptomatic orthostatic hypotension
  8. Estimated glomerular filtration rate is at least 60 mL/Min/1.73m<sup>2</sup>
  9. Will not be used in combination with or alternating growth hormone, growth hormone analog, or Increlex (mecasermin)

**Initial approval duration:** 6 months

- **Criteria for continuation of coverage (renewal request):** Voxzogo (vosoritide) is considered *medically necessary* and will be approved when **ALL** of the following criteria are met:
1. Individual continues to be seen by a physician specializing in the patient's diagnosis or is in consultation with an Endocrinologist or Pediatric Endocrinologist
  2. Individual's condition has responded while on therapy
    - a. Response is defined as:
      - i. Growth velocity is at least 1.5 cm/year
      - ii. No evidence of disease progression
      - iii. No evidence individual has developed any significant unacceptable adverse drug reactions that may exclude continued use



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3. Recent (within the last 12 months) radiographic evidence epiphyses have not closed
4. Individual has been adherent with the medication
5. Individual does not have hypochondroplasia or short stature condition other than achondroplasia
6. Individual is not planning to have limb-lengthening surgery
7. Individual does not have systolic blood pressure (BP) < 70 millimeters of mercury (mm Hg) or recurrent symptomatic hypotension (defined as episodes of low BP generally accompanied by symptoms such as, dizziness, fainting) or recurrent symptomatic orthostatic hypotension
8. Estimated glomerular filtration rate is at least 60 mL/Min/1.73m<sup>2</sup>
9. Will not be used in combination with or alternating growth hormone, growth hormone analog, or Increlex (mecasermin)

**Renewal duration:** 12 months

➤ Criteria for a request for non-FDA use or indication, treatment with dosing, frequency, or duration outside the FDA-approved dosing, frequency, and duration, refer to one of the following Pharmacy Coverage Guideline:

1. **Off-Label Use of Non-cancer Medications**
2. **Off-Label Use of Cancer Medications**

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### **Description:**

Voxzogo (vosoritide) is a C type natriuretic peptide (CNP) analog 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).

In patients with achondroplasia, endochondral bone growth is negatively regulated due to a gain of function mutation in fibroblast growth factor receptor 3 (*FGFR3*). Binding of vosoritide to natriuretic peptide receptor-B (NPR-B) antagonizes *FGFR3* downstream signaling by inhibiting the extracellular signal-regulated kinases 1 and 2 (ERK1/2) in the mitogen-activated protein kinase (MAPK) pathway at the level of rapidly accelerating fibrosarcoma serine/threonine protein kinase (RAF-1). As a result, vosoritide, like CNP, acts as a positive regulator of endochondral bone growth as it promotes chondrocyte proliferation and differentiation.



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In animal models with open growth plates, vosoritide administration resulted in the promotion of chondrocyte proliferation and differentiation that led to a widening of the growth plate and subsequent increase in skeletal growth. In models of FGFR3-related chondrodysplasia, a partial or complete normalization of the dwarfism phenotype was observed.

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### **Resources:**

Voxzogo (vosoritide) product information, revised by BioMarin Pharmaceutical Inc. 11-2021. Available at DailyMed <http://dailymed.nlm.nih.gov>. Accessed January 31, 2022.

Bacino CA. Achondroplasia. In: UpToDate, Hahn S, TePas E (Eds), UpToDate, Waltham MA.: UpToDate Inc. Available at <http://uptodate.com>. Topic last updated January 07, 2022. Accessed February 07, 2022.

ClinicalTrials.gov Identifier NCT03197766. A study to evaluate the efficacy and safety of BMN 111 in children with achondroplasia. Last Updated May 15, 2020. Available from: <http://clinicaltrials.gov>. Accessed February 07, 2022.