

vosoritide (Voxzogo™)**Policy # 00787**

Original Effective Date: 06/13/2022

Current Effective Date: 06/01/2026

Applies to all products administered or underwritten by Blue Cross and Blue Shield of Louisiana and its subsidiary, HMO Louisiana, Inc. (collectively referred to as the "Company"), unless otherwise provided in the applicable contract. Medical technology is constantly evolving, and we reserve the right to review and update Medical Policy periodically.

Investigational or experimental services are not covered. This includes any drug, device, procedure, or service provided under the investigational arm of a clinical trial or clinical study. These services are excluded from coverage under benefits.

When Services May Be Eligible for Coverage

Coverage for eligible medical treatments or procedures, drugs, devices or biological products may be provided only if:

- *Benefits are available in the member's contract/certificate, and*
- *Medical necessity criteria and guidelines are met.*

Based on review of available data, the Company may consider vosoritide (Voxzogo™)[†] for the treatment of patients with achondroplasia to be **eligible for coverage**.^{**}

Patient Selection Criteria

Coverage eligibility for the use of vosoritide (Voxzogo) will be considered when the following criteria are met:

Initial:

- Requested drug is used to increase linear growth due to achondroplasia, which has been confirmed by genetic testing showing an identifiable mutation in the fibroblast growth factor receptor type 3 (FGFR3) gene; AND
- Patient is younger than 18 years of age; AND
- Patient has open epiphyses; AND
- Patient's annualized growth velocity is greater than or equal to 1.5 centimeters per year; AND

*(Note: This specific patient selection criterion is an additional Company requirement, based on clinical trials, for coverage eligibility and will be denied as not medically necessary** if not met).*

- Patient is able to drink approximately 240 mL to 300 mL of fluid in the hour prior to Voxzogo administration; AND
- Patient is NOT expected to have limb-lengthening surgery while on therapy with Voxzogo AND has NOT previously had limb-lengthening surgery.

*(Note: This specific patient selection criterion is an additional Company requirement, based on clinical trials, for coverage eligibility and will be denied as not medically necessary** if not met).*

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Continuation

- Patient has received initial approval for the requested drug; AND
- Patient is younger than 18 years of age; AND
- Patient has open epiphyses; AND
- Patient's annualized growth velocity is greater than or equal to 1.5 centimeters per year; AND
*(Note: This specific patient selection criterion is an additional Company requirement, based on clinical trials, for coverage eligibility and will be denied as not medically necessary** if not met).*
- Patient is able to drink approximately 240 mL to 300 mL of fluid in the hour prior to Voxzogo administration; AND
- Patient is NOT expected to have limb-lengthening surgery while on therapy with Voxzogo AND has NOT previously had limb-lengthening surgery; AND
*(Note: This specific patient selection criterion is an additional Company requirement, based on clinical trials, for coverage eligibility and will be denied as not medically necessary** if not met).*
- Patient's most recent annualized growth velocity is above their baseline annualized growth velocity (that is, before the patient started on Voxzogo).
*(Note: This specific patient selection criterion is an additional Company requirement for coverage eligibility and will be denied as not medically necessary** if not met).*

When Services Are Considered Not Medically Necessary

Based on review of available data, the Company considers the use of vosoritide (Voxzogo) if the patient's growth velocity is less than 1.5 centimeters per year to be **not medically necessary.****

Based on review of available data, the Company considers the use of vosoritide (Voxzogo) if there are plans to have limb lengthening surgery while on therapy with the requested drug to be **not medically necessary.****

Based on review of available data, the Company considers the use of vosoritide (Voxzogo) if the patient had limb-lengthening surgery in the past to be **not medically necessary.****

Based on review of available data, the Company considers the continued use of vosoritide (Voxzogo) if the patient has NOT responded to therapy with the requested medication to be **not medically necessary.****

When Services Are Considered Investigational

Coverage is not available for investigational medical treatments or procedures, drugs, devices or biological products.

Based on review of available data, the Company considers the use of vosoritide (Voxzogo) when patient selection criteria are not met (except the criterion denoted above as **not medically necessary****) to be **investigational.***

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Background/Overview

Voxzogo is a C type natriuretic peptide (CNP) analog indicated to increase linear growth in pediatric patients with achondroplasia with open epiphyses. Voxzogo is administered once daily via subcutaneous injection and the dosing is weight based. See the package insert for specific dosing information. Voxzogo is supplied as 0.4 mg, 0.56 mg, and 1.2 mg lyophilized powder in single dose vials for reconstitution. Patients need to ensure adequate food and fluid intake prior to administration. Specifically, the package insert notes that patients need to drink approximately 240-300 mL of fluid in the hour prior to Voxzogo administration.

Achondroplasia is the most common bone dysplasia in humans. Clinical manifestations include disproportionate short stature, long-bone shortening, and macrocephaly. Achondroplasia is caused by pathogenic variants in the fibroblast growth factor receptor 3 (*FGFR3*) gene. This variant permanently activates the *FGFR3* receptor. This activation inhibits chondrocyte proliferation, therefore leading to impaired bone growth. CNP works by inhibiting downstream signaling produced by the overactive *FGFR3* gene. This downstream inhibition allows for chondrocyte proliferation and differentiation, thereby leading to bone formation. The administration of Voxzogo provides the CNP analog, which works to inhibit the effects of the overactive *FGFR3* gene. Complications associated with achondroplasia include recurrent otitis media, sleep-disordered breathing, obesity, leg bowing, narrowing of the lumbar spine, and cervical medullary compression. Prior to the approval of Voxzogo, there were no FDA approved drugs for the treatment of this condition.

FDA or Other Governmental Regulatory Approval

U.S. Food and Drug Administration (FDA)

Voxzogo is indicated to increase linear growth in pediatric patients with achondroplasia who are 5 years of age and older with open epiphyses. In October 2023, the FDA label was expanded to include all pediatric patients.

Rationale/Source

This medical policy was developed through consideration of peer-reviewed medical literature generally recognized by the relevant medical community, U.S. Food and Drug Administration approval status, nationally accepted standards of medical practice and accepted standards of medical practice in this community, technology evaluation centers, reference to federal regulations, other plan medical policies, and accredited national guidelines.

The safety and effectiveness of Voxzogo in patients with achondroplasia were assessed in one 52-week, multi-center, randomized, double-blind, placebo-controlled, phase 3 study - Study 1. Study 1 was conducted in 121 subjects with genetically-confirmed achondroplasia, who were randomized to either Voxzogo (n=60) or placebo (n=61). The dosage of Voxzogo was 15 mcg/kg administered subcutaneously once daily. Baseline standing height, weight Z-score, body mass index (BMI) Z-score, and upper to lower body ratio were collected for at least 6 months prior to randomization. Subjects with limb-lengthening surgery in the prior 18 months or who planned to have limb-lengthening surgery during the study period were excluded. Subjects with a growth velocity less than 1.5 centimeters per year were excluded from the study. The study included a 52-week placebo-controlled treatment phase followed by an open-label treatment extension study period in which all

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subjects received Voxzogo. The primary efficacy endpoint was the change from baseline in annualized growth velocity (AGV) at Week 52 compared with placebo. The subjects' ages ranged from 5.1 to 14.9 years with a mean of 8.7 years. The subjects had a mean baseline height standard deviation score (SDS) of -5.13. Treatment with Voxzogo for 52 weeks resulted in a treatment difference in the change from baseline in AGV of 1.57 cm/year after 52 weeks of treatment.

References

1. Voxzogo [package insert]. BioMarin Pharmaceuticals, Inc. Novato, California. Updated October 2023.

Policy History

Original Effective Date: 06/13/2022

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05/05/2022	Medical Policy Committee review
05/11/2022	Medical Policy Implementation Committee approval. New policy.
05/04/2023	Medical Policy Committee review
05/10/2023	Medical Policy Implementation Committee approval. Coverage eligibility unchanged.
05/02/2024	Medical Policy Committee review
05/08/2024	Medical Policy Implementation Committee approval. Updated age requirement to reflect FDA label expansion to all pediatric patients.
05/01/2025	Medical Policy Committee review
05/13/2025	Medical Policy Implementation Committee approval. Coverage eligibility unchanged.
05/07/2026	Medical Policy Committee review
05/13/2026	Medical Policy Implementation Committee approval. Coverage eligibility unchanged.

Next Scheduled Review Date: 05/2027

*Investigational – A medical treatment, procedure, drug, device, or biological product is Investigational if the effectiveness has not been clearly tested and it has not been incorporated into standard medical practice. Any determination we make that a medical treatment, procedure, drug, device, or biological product is Investigational will be based on a consideration of the following:

- A. Whether the medical treatment, procedure, drug, device, or biological product can be lawfully marketed without approval of the U.S. Food and Drug Administration (FDA) and whether such approval has been granted at the time the medical treatment, procedure, drug, device, or biological product is sought to be furnished; or
- B. Whether the medical treatment, procedure, drug, device, or biological product requires further studies or clinical trials to determine its maximum tolerated dose, toxicity, safety, effectiveness, or effectiveness as compared with the standard means of treatment or diagnosis, must improve health outcomes, according to the consensus of opinion among experts as shown by reliable evidence, including:
 1. Consultation with technology evaluation center(s);

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2. Credible scientific evidence published in peer-reviewed medical literature generally recognized by the relevant medical community; or
3. Reference to federal regulations.

****Medically Necessary (or “Medical Necessity”)** - Health care services, treatment, procedures, equipment, drugs, devices, items or supplies that a Provider, exercising prudent clinical judgment, would provide to a patient for the purpose of preventing, evaluating, diagnosing or treating an illness, injury, disease or its symptoms, and that are:

- A. In accordance with nationally accepted standards of medical practice;
- B. Clinically appropriate, in terms of type, frequency, extent, level of care, site and duration, and considered effective for the patient's illness, injury or disease; and
- C. Not primarily for the personal comfort or convenience of the patient, physician or other health care provider, and not more costly than an alternative service or sequence of services at least as likely to produce equivalent therapeutic or diagnostic results as to the diagnosis or treatment of that patient's illness, injury or disease.

For these purposes, “nationally accepted standards of medical practice” means standards that are based on credible scientific evidence published in peer-reviewed medical literature generally recognized by the relevant medical community, Physician Specialty Society recommendations and the views of Physicians practicing in relevant clinical areas and any other relevant factors.

‡ Indicated trademarks are the registered trademarks of their respective owners.

NOTICE: If the Patient’s health insurance contract contains language that differs from the BCBSLA Medical Policy definition noted above, the definition in the health insurance contract will be relied upon for specific coverage determinations.

NOTICE: Medical Policies are scientific based opinions, provided solely for coverage and informational purposes. Medical Policies should not be construed to suggest that the Company recommends, advocates, requires, encourages, or discourages any particular treatment, procedure, or service, or any particular course of treatment, procedure, or service.

NOTICE: Federal and State law, as well as contract language, including definitions and specific contract provisions/exclusions, take precedence over Medical Policy and must be considered first in determining eligibility for coverage.

NOTICE: If an authorization for an ongoing course of treatment has been provided to a member and the member changes from one health plan to another health plan (e.g., a member moves from carrier A to Louisiana Blue), Louisiana Blue may honor the previous health plan’s authorization for the same service under the same type of in-network benefit for a 90-day transition period. Documentation of the authorization for the ongoing course of treatment from the previous health plan must be provided to us by the member or their provider and the services provided for the course of treatment must otherwise be a covered service under the Louisiana Blue health plan. This provision does not apply to medications covered under the plan’s pharmacy benefit.