Pharmacy Policy Bulletin

Title: Vosoritide (Voxzogo™)

Policy #: Rx.01.260

Application of pharmacy policy is determined by benefits and contracts. Benefits may vary based on product line, group, or contract. Some medications may be subject to precertification, age, quantity, or formulary restrictions (ie limits on non-preferred drugs). Individual member benefits must be verified.

This pharmacy policy document describes the status of pharmaceutical information and/or technology at the time the document was developed. Since that time, new information relating to drug efficacy, interactions, contraindications, dosage, administration routes, safety, or FDA approval may have changed. This Pharmacy Policy will be regularly updated as scientific and medical literature becomes available. This information may include new FDA-approved indications, withdrawals, or other FDA alerts. This type of information is relevant not only when considering whether this policy should be updated, but also when applying it to current requests for coverage.

Members are advised to use participating pharmacies in order to receive the highest level of benefits.

Intent:

The intent of this policy is to communicate the medical necessity criteria for **Vosoritide** (**Voxzogo™**) as provided under the member's prescription drug benefit.

Description:

Achondroplasia is the most common bone dysplasia in humans, with a prevalence of approximately 1 in 20,000 live births. It is an autosomal dominant condition caused by pathogenic variants in the fibroblast growth factor receptor 3 (FGFR3) gene. The most salient clinical features include disproportionate short stature (adult height is approximately 4 feet), long-bone shortening that predominantly affects the proximal aspects of the upper and lower extremities (rhizomelic shortening), and macrocephaly. Patients with achondroplasia may have delayed motor development early on, but cognition is normal. Use of growth hormone is not recommended and can potentially worsen the disproportion seen in these patients.

Vosoritide (Voxzogo™) is indicated to increase linear growth in pediatric patients with achondroplasia who are 5 years of age and older with open epiphyses.

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.

Policy:

INITIAL CRITERIA: Vosoritide (Voxzogo™) is approved when ALL of the following are met:

1. Diagnosis of achondroplasia as confirmed by ONE of the following:
    a. BOTH of the following:
        i. Member has clinical manifestations characteristic of achondroplasia (e.g., macrocephaly, frontal bossing, midface retrusion, disproportionate short stature with rhizomelic shortening of the arms and the legs, brachydactyly, trident configuration of the hands, thoracolumbar kyphosis, and accentuated lumbar lordosis); and
        ii. Member has radiographic findings characteristic of achondroplasia (e.g., large calvaria and narrowing of the foramen magnum region, undertubulated, shortened long bones with metaphyseal abnormalities, narrowing of the interpedicular distance of the caudal spine, square ilia and horizontal acetabula, small sacrosciatic notches, proximal scooping of the femoral metaphyses, and short and narrow chest); or
    b. Molecular genetic testing confirmed c.1138G>A or c.1138G>C variant (i.e., p.Gly380Arg mutation) in the fibroblast growth factor receptor-3 (FGFR3) gene; and
2. Member has open epiphyses; and
3. Member is 5 years of age or older; and
4. Prescribed by or in consultation with one of the following:

a. Clinical geneticist; or
b. Endocrinologist; or
c. A provider who has specialized expertise in the management of achondroplasia

Initial authorization duration: 12 months

REAUTHORIZATION CRITERIA: Vosoritide (Voxzogo™) is re-approved when ALL of the following are met:

1. Member continues to have open epiphyses; and
2. Documentation of positive clinical response to therapy [e.g., improvement in annualized growth velocity (AGV) compared to baseline]; and
3. Prescribed by or in consultation with one of the following:
   a. Clinical geneticist; or
   b. Endocrinologist; or
   c. A provider who has specialized expertise in the management of achondroplasia

Reauthorization duration: 12 months

Black Box Warning as shown in the drug Prescribing Information:

N/A

Guidelines:

Refer to the specific manufacturer's prescribing information for administration and dosage details and any applicable Black Box warnings.

BENEFIT APPLICATION

Subject to the terms and conditions of the applicable benefit contract, the applicable drug(s) identified in this policy is (are) covered under the prescription drug benefits of the Company's products when the medical necessity criteria listed in this pharmacy policy are met. Any services that are experimental/investigational or cosmetic are benefit contract exclusions for all products of the Company.

References:

Achondroplasia. UpToDate. January 2022. Available at: https://www.uptodate.com/contents/achondroplasia?search=achondroplasia&source=search_result&selectedTitle=1~89&usage_type=default&display_rank=1. Accessed February 01, 2023.

Vosoritide (voxzogo) [package insert]. Novato, CA. BioMarin Pharmaceutical Inc. December 2021. Available at: https://dailymed.nlm.nih.gov/dailymed/drugInfo.cfm?setid=228e8560-04a4-4bb1-a81f-29531a9e4d27. Accessed February 01, 2023.

Applicable Drugs:

Inclusion of a drug in this table does not imply coverage. Eligibility, benefits, limitations, exclusions, precertification/referral requirements, provider contracts, and Company policies apply.

Brand Name	Generic Name
Voxzogo™	Vosoritide

Cross References:

Rx.01.33 Off Label Use

Policy Version Number:	2.00
P&T Approval Date:	December 08, 2022
Policy Effective Date:	April 01, 2023
Next Required Review Date:	December 08, 2023

The Policy Bulletins on this web site were developed to assist the Company in administering the provisions of the respective benefit programs, and do not constitute a contract. If you have coverage through the Company, please refer to your specific benefit program for the terms, conditions, limitations and exclusions of your coverage. Company does not provide health care services, medical advice or treatment, or guarantee the outcome or results of any medical services/treatments. The facility and professional providers are responsible for providing medical advice and treatment. Facility and professional providers are independent contractors and are not employees or agents of the Company. If you have a specific medical condition, please consult with your doctor. The Company reserves the right at any time to change or update its Policy Bulletins.