Policy and Procedure	
PHARMACY PRIOR AUTHORIZATION POLICY AND CRITERIA ORPTCEND076.0425	**ENDOCRINE & METABOLIC DRUGS** **VOXZOGO®** (vosoritide for subcutaneous injection)
Effective Date: 6/1/2025	**Review/Revised Date**: 01/23, 02/24, 03/24, 02/25 (JH)
Original Effective Date: 06/22	**P&T Committee Meeting Date**: 04/22, 04/23, 02/24, 04/24, 04/25
Approved by: Oregon Region Pharmacy and Therapeutics Committee	

SCOPE:
Providence Health Plan and Providence Health Assurance as applicable (referred to individually as "Company" and collectively as "Companies").

APPLIES TO:
Commercial
Medicaid

POLICY CRITERIA:

COVERED USES:
All Food and Drug Administration (FDA)-Approved Indications

REQUIRED MEDICAL INFORMATION:
For Medicaid: Coverage is limited to a condition that has been designated a covered line item number by the Oregon Health Services Commission listed on the Prioritized List of Health Care Services

For initial authorization, ALL the following criteria must be met:
1. Documentation of confirmed diagnosis of achondroplasia (Q77.4) through genetic testing
   AND
2. Documentation of a baseline annual growth velocity (AGV)
   AND
3. Current annual growth velocity greater than or equal to 1.5 cm/year (0.6 in/year)
   AND
4. Evidence of open epiphyses, defined as follows:
   a. Tanner stage less than 4
      OR
   b. Bone age less than 16 years in male or less than 14 years in female. Bone age must be obtained annually when chronologic age reaches 15 years in male or 13 years in female

   AND
5. Person is ambulatory and able to stand without assistance

For reauthorization, ALL of the following criteria must be met:

1. Documentation of *an improvement* in annual growth velocity of greater than or equal to 1.0 cm/year from baseline (for example, if baseline AGV is 2.0 cm/year, 3.0 cm/year is required for reauthorization)
   AND
2. Current growth velocity greater than or equal to 1.5 cm/year (0.6 in/year)
   AND
3. One of the following:
   a. Tanner stage less than 4
      OR
   b. Bone age less than 16 years in male or less than 14 years in female. Bone age must be obtained annually when chronologic age reaches 15 years in male or 13 years in female

   AND
4. Person is ambulatory and able to stand without assistance

EXCLUSION CRITERIA:

- History of bone-related surgery or fracture of long bone or spine within the previous six months
- Planned bone surgery

AGE RESTRICTIONS: N/A

PRESCRIBER RESTRICTIONS:
Must be prescribed by, or in consultation with, a pediatric endocrinologist or other prescriber specialized in the care of patients with achondroplasia or skeletal dysplasia.

COVERAGE DURATION:
Initial authorization and reauthorization will be approved for one year. Shorter reauthorization period may be approved based on slowing of growth velocity or bone age approaching epiphyseal closure.

QUANTITY LIMIT:
One vial per day

Requests for indications that were approved by the FDA within the previous six (6) months may not have been reviewed by the health plan for safety and effectiveness and inclusion on this policy document. These requests will be reviewed using the New Drug and or Indication Awaiting P&T Review; Prior Authorization Request ORPTCOPS047.

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Requests for a non-FDA approved (off-label) indication requires the proposed indication be listed in either the American Hospital Formulary System (AHFS), Drugdex, or the National Comprehensive Cancer Network (NCCN) and is considered subject to evaluation of the prescriber's medical rationale, formulary alternatives, the available published evidence-based research and whether the proposed use is determined to be experimental/investigational.

Coverage for Medicaid is limited to a condition that has been designated a covered line item number by the Oregon Health Services Commission listed on the Prioritized List of Health Care Services.

Coverage decisions are made on the basis of individualized determinations of medical necessity and the experimental or investigational character of the treatment in the individual case.

INTRODUCTION:

Vosoritide (Voxzogo®) is a self-injectable medication used to increase height in children with a genetic disorder called achondroplasia (ACH). Achondroplasia impairs the growth of bone in the limbs, causes abnormal growth in the spine and skull, and leads to disproportionate short stature. In individuals with ACH, the average adult height of a male is 132 cm (4ft 4in), and female is 124 cm (4 ft 1in).

FDA APPROVED INDICATIONS:

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).

POSITION STATEMENT:

- Achondroplasia is the most common bone dysplasia, with a prevalence of approximately 1 in 40,000 live births. It is an autosomal dominant condition caused by genetic mutation in the fibroblast growth factor receptor 3 (FGFR3) gene. However, only about one out of five cases is hereditary and the rest are the result of new (de novo) pathogenic variants.
- The diagnosis of ACH can be based upon clinical manifestations and radiographic findings but must be confirmed by targeted genetic testing for the classical 1138 variant in the FGFR3 genes. Other differential diagnoses that share similar clinical and/or radiologic features as ACH include hypochondroplasia, thanatophoric dysplasia and homozygous achondroplasia.
- Achondroplasia can lead to a number of medical complications such as recurrent otitis media, sleep apnea, leg bowing, spinal stenosis and cervicomedullary compression. In severe cases, surgical intervention may be necessary.

- The use of growth hormone is not recommended because it may worsen the disproportionate stature.
- Vosoritide (Voxzogo®) is the first medication approved by the FDA for the treatment of ACH. It is a recombinant C-type natriuretic peptide analog that works by regulating the signaling pathway downstream of the fibroblast growth factor receptor 3 (FGFR3) gene to assist bone growth.
- The safety and efficacy of vosoritide SQ injection in ACH was based on one phase 3, 52-wk, randomized, double-blinded, placebo-controlled study in 121 subjects between 5.1-14.9 years (mean 8.7) that showed an improved annualized growth velocity (AGV) of 1.57 cm/yr vs. placebo and improvement of AGV was maintained for the 2-year duration at follow-up.
- The most common adverse reactions (>10%) associated with vosoritide SQ injection are injection site erythema, injection site swelling, vomiting, injection site urticaria, arthralgia, decreased blood pressure, and gastroenteritis. There is also warning for transient decrease in blood pressure.

REFERENCE/RESOURCES:

1. Voxzogo® monograph. Prime Therapeutics. Created on Nov. 19, 2021.
2. Voxzogo® Package Insert. Novato, CA: BioMarin Pharmaceutical Inc. Nov 2024
3. Voxzogo® In: DRUGDEX® System [Internet database]. Greenwood Village, Colo: Thomson Reuters (Healthcare) Inc. Updated periodically.
4. Voxzogo® In: Lexi-Drugs Online [Internet database]. Hudson, OH: Lexi-Comp, Inc. Updated periodically.
5. Achondroplasia. Johns Hopkins Medicine. Available at: https://www.hopkinsmedicine.org/health/conditions-and-diseases/achondroplasia. Accessed March 3, 2022
6. ClinicalTrials.gov [Internet]. Bethesda (MD): National Library of Medicine (US). 2000 Feb 29 - . Identifier NCT03197766, A Study to Evaluate the Efficacy and Safety of BMN 111 in Children With Achondroplasia. Available from: https://clinicaltrials.gov/ct2/show/NCT03197766?term=NCT03197766&draw=2&rank=1
7. Bacino CA (2022). Achondroplasia. UptoDate. Available at: https://www.uptodate.com/contents/achondroplasia?search=achondroplasia%20treatment&source=search_result&selectedTitle=1~150&usage_type=default&display_rank=1
8. Achondroplasia growth charts for boys. Available at : https://www.achondroplasia-growthcharts.com/Achondroplasia-charts-booklet-boys.pdf

9. Achondroplasia growth charts for girls. Available at: https://www.achondroplasia-growthcharts.com/Achondroplasia-charts-booklet-girls.pdf
10. Coste J, Pouchot J, Carel JC. Height and Health-Related Quality of Life: A Nationwide Population Study. The Journal of Clinical Endocrinology & Metabolism, Volume 97, Issue 9, 1 September 2012, Pages 3231–3239, https://doi.org/10.1210/jc.2012-1543