PRIOR AUTHORIZATION POLICY

POLICY: Growth Disorders – Voxzogo Prior Authorization Policy
- Voxzogo™ (vosoritide subcutaneous injection – BioMarin)

REVIEW DATE: 11/20/2024

OVERVIEW
Voxzogo, a C type natriuretic peptide (CNP) analog, is indicated **to increase linear growth in pediatric patients with achondroplasia** with open epiphyses.[1]

Disease Overview
Achondroplasia is the most common form of disproportionate short stature in humans.[2] It is a primary skeletal dysplasia caused by a mutation in the fibroblast growth factor receptor 3 (FGFR3) gene; this mutation leads to impaired endochondral ossification. Achondroplasia occurs in approximately 1 in 20,000 to 30,000 live births.[3] It occurs as a result of a spontaneous mutation in 80% of patients (i.e., both parents are of normal height).[4] In the remaining 20% of patients, the mutation is inherited from a parent. Achondroplasia is characterized by short stature, long-bone shortening in the proximal upper and lower extremities, and macrocephaly. The diagnosis can be confirmed by molecular testing.[5] In the pivotal trial for Voxzogo, achondroplasia was confirmed by genetic testing in all patients.[2] Additionally, exclusion criteria included evidence of decreased growth velocity (< 1.5 cm/year) or of growth plate closure through bilateral lower extremity X-rays.

POLICY STATEMENT
Prior Authorization is recommended for prescription benefit coverage of Voxzogo. All approvals are provided for the duration noted below. Because of the specialized skills required for evaluation and diagnosis of patients treated with Voxzogo as well as the monitoring required for adverse events and long-term efficacy, approval requires Voxzogo to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA
Coverage of Voxzogo is recommended in those who meet the following criteria:

FDA-Approved Indication

1. **Achondroplasia.** Approve for 1 year if the patient meets ONE of the following (A or B):
   - **A)** Initial Therapy or Patient Has Been on Voxzogo for < 1 Year. Approve if the patient meets ALL of the following (i, ii, iii, iv, v, and vi):
     - **i.** Patient is < 18 years of age; AND
     - **ii.** The diagnosis of achondroplasia has been confirmed by genetic testing with an identifiable mutation in the fibroblast growth factor receptor type 3 (FGFR3) gene; AND
     - **iii.** Patient's epiphyses are open; AND
     - **iv.** Patient will not have limb-lengthening surgery during treatment with Voxzogo; AND
     - **v.** The prescriber has confirmed the patient is able to drink approximately 240 to 300 mL of fluid in the hour prior to Voxzogo administration; AND

vi. The medication is prescribed by or in consultation with a pediatric endocrinologist.

B) Patient Has Been Receiving Voxzogo for ≥ 1 Year. Approve if the patient meets ALL of the following (i, ii, iii, iv, v, vi, and vii):
- **i.** Patient is < 18 years of age; AND
- **ii.** The diagnosis of achondroplasia has been confirmed by genetic testing with an identifiable mutation in the fibroblast growth factor receptor type 3 (FGFR3) gene; AND
- **iii.** Patient's epiphyses are open; AND
- **iv.** Patient will not have limb-lengthening surgery during treatment with Voxzogo; AND
- **v.** The prescriber has confirmed the patient is able to drink approximately 240 to 300 mL of fluid in the hour prior to Voxzogo administration; AND
- **vi.** The medication is prescribed by or in consultation with a pediatric endocrinologist; AND
- **vii.** Patient's most recent annualized growth velocity continues to be above their baseline annualized growth velocity value (i.e., before the patient started on Voxzogo).

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Voxzogo is not recommended in the following situations:

1. **Hypochondroplasia, Thanatophoric Dysplasia, or other Short Stature Conditions other than Achondroplasia (e.g., Trisomy 21, Pseudoachondroplasia).** Voxzogo is only indicated for patients with achondroplasia.[1] There is a small published Phase 2 trial showing some efficacy for children with hypochondroplasia.[11] There is no evidence Voxzogo is effective for other short stature conditions.

2. **Concurrent Treatment with Growth Hormone (e.g., somatropin), Long-Acting Growth Hormone (e.g., Ngenla® [somatrogon-ghla], Skytrofa® [lonapegsomatropin], Sogroya® [somapacitan-beco]), or Insulin-like Growth Factor- 1 (IGF-1) [i.e., Increlex® {mecasermin}] Agents.** Growth hormone agents and Increlex are NOT indicated to increase growth in patients with achondroplasia.[6-10] Additionally, there are no available studies demonstrating the safety or efficacy of concurrent use with Voxzogo.

3. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

REFERENCES

1. Voxzogo™ subcutaneous injection [prescribing information]. Novato, CA: BioMarin; November 2024.
2. Once-daily, subcutaneous vosoritide therapy in children with achondroplasia: a randomised, double-blind, phase 3, placebo-controlled, multicentre trial. *Lancet.* 2020;396(10252):684-692.
3. National Organization for Rare Disorders (NORD). Achondroplasia Last updated November 17, 2023. Available at: Achondroplasia - NORD (National Organization for Rare Disorders) (rarediseases.org). Accessed on November 18, 2024.
4. Achondroplasia: a comprehensive clinical disease. *Orphanet J Rare Dis*. 2019;14(1):1.
5. Health supervision for people with achondroplasia. American Academy of Pediatrics. *Pediatrics*. 2020;145(6):e20201010.
6. Norditropin® subcutaneous injection [prescribing information]. Plainsboro, NJ: Novo Nordisk; March 2020.
7. Skytrofa™ subcutaneous injection [prescribing information]. Princeton, NJ: Ascendis Pharma; May 2024.
8. Sogroya® subcutaneous injection [prescribing information]. Plainsboro, NJ: Novo Nordisk; April 2023.
9. Ngenla® subcutaneous injection [prescribing information]. New York, NY: Pfizer; June 2023.
10. Increlex® subcutaneous injection [prescribing information]. Cambridge, MA: Ipsen; March 2024.
11. Dauber A, Zhang A, Kanakatti Shankar R, et al. Vosoritide treatment for children with hypochondroplasia: a phase 2 trial. *EClinicalMedicine*. 2024;71:102591.