Reference number(s)
5069-A

Specialty Guideline Management
Voxzogo

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Voxzogo	vosoritide

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications[1]

Voxzogo is indicated 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).

All other indications are considered experimental/investigational and not medically necessary.

Documentation

Submission of the following information is necessary to initiate the prior authorization review:

- Chart notes or documentation of symptoms (i.e., short stature with marked shortening of extremities due to rhizomelia, a characteristic facial configuration, trident hand) (if applicable)
- Laboratory report of X-ray findings consistent with achondroplasia or laboratory report of genetic testing for FGFR3 mutation
- Growth chart
- For continuation requests: Chart notes or medical record documentation confirming benefit from therapy (e.g., growth chart showing improvement or stabilization of annualized growth velocity [centimeters per year])

Prescriber Specialties

This medication must be prescribed by or in consultation with an endocrinologist, pediatric endocrinologist, geneticist, or neurologist.

Coverage Criteria

Achondroplasia

Authorization of 12 months may be granted for treatment of achondroplasia when BOTH of the following criteria are met:

- The diagnosis of achondroplasia was confirmed by EITHER of the following:
    - Symptoms (i.e., short stature with marked shortening of extremities due to rhizomelia, a characteristic facial configuration, trident hand) AND X-ray findings consistent with achondroplasia
    - Genetic testing for FGFR3 mutation
- Epiphyses are open

Continuation of Therapy

Authorization of 12 months may be granted for continuation of therapy when BOTH of the following criteria are met:

- Member meets all requirements in the coverage criteria
- Member is experiencing benefit from therapy (e.g., improvement or stabilization of annualized growth velocity [centimeters per year] from baseline)

References

1. Voxzogo [package insert]. Novato, CA: BioMarin Pharmaceutical Inc.; November 2024.

2. Kubota T, Adachi M, Kitaoka T, et al. Clinical Practice Guidelines for Achondroplasia. Clin Pediatr Endocrinol. 2020;29(1):25-42.
3. Trotter TL, Hall JG, American Academy of Pediatrics Committee on Genetics. Health supervision for children with achondroplasia. Pediatrics. 2005;116(3):771–783.
4. Hoover-Fong J, Scott CI, Jones MC, Committee on Genetics. Health supervision for people with achondroplasia. Pediatrics. 2020;145(6):e20201010.