



Support their bone growth as early as birth

The first and only treatment FDA approved from birth until growth plates close

Sophie

9 MONTHS OLD
on VOXZOGO since
3 months old

VOXZOGO[®]
(vosoritide) for injection

Indicated to increase linear growth in infants and children with achondroplasia.¹

INDICATION AND IMPORTANT SAFETY INFORMATION

VOXZOGO[®] (vosoritide) is indicated to increase linear growth in pediatric patients with achondroplasia and open growth plates.

- 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).

Warnings and Precautions for Risk of Low Blood Pressure

Transient decreases in blood pressure were observed in clinical studies. Patients with significant cardiac or vascular disease and patients on anti-hypertensive medicinal products were excluded from participation in VOXZOGO clinical trials. To reduce the risk of a decrease in blood pressure and associated symptoms (dizziness, fatigue, and/or nausea), patients should be well hydrated, have adequate food intake, and drink approximately 8-10 ounces of fluid in the hour prior to VOXZOGO administration.

Please see additional Important Safety Information presented throughout, and in the full [Prescribing Information](#).

You can make a difference from day 1

Support better management of achondroplasia with proactive and multidisciplinary care starting at birth^{2,3}

Achondroplasia is the most common type of skeletal dysplasia, a rare genetic condition that is **often diagnosed prenatally or at birth**.³⁻⁵

Helping families navigate an achondroplasia diagnosis^{2,3,6}



Congratulate and reassure parents as they welcome a new child to their family while helping them to feel optimistic about the prognosis through comprehensive healthcare, community, and family support.^{2,3}



Educate on achondroplasia including potential health complications along with steps they can take to support and care for their child from day 1.^{2,3}



Inform about the care pathway ahead by outlining relevant specialists, the importance of timely intervention, and treatment options available from birth to help prepare for postnatal management.^{2,3,6}



Connect to specialized care quickly through referrals to healthcare providers experienced in achondroplasia care.^{2,3}

IMPORTANT SAFETY INFORMATION

Warnings and Precautions for Risk of Low Blood Pressure (cont'd)

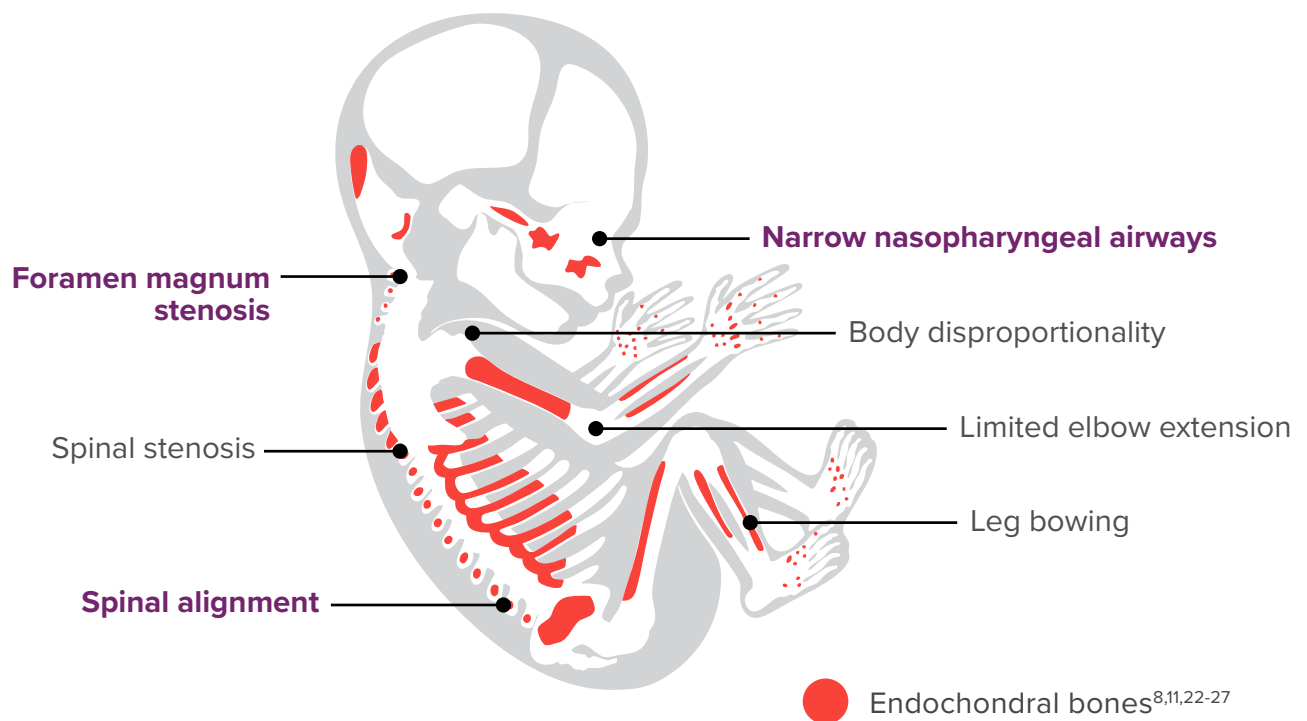
In a 52-week, randomized, double-blind, placebo-controlled trial in 121 subjects with achondroplasia, subjects aged from 5.1 to 14.9 years, (Study 1) eight (13%) of 60 patients treated with VOXZOGO had a total of 11 events of transient decrease in blood pressure, compared to 3 (5%) of 61 patients on placebo, over a 52-week treatment period. The median time to onset from injection was 31 (18 to 120) minutes, with resolution within 31 (5 to 90) minutes in VOXZOGO-treated subjects. Two out of 60 (3%) VOXZOGO-treated patients each had one symptomatic episode of decreased blood pressure with vomiting and/or dizziness compared to 0 of 61 (0%) patients on placebo.

Endochondral bone growth is inhibited throughout the body in achondroplasia^{3,7-9}

Achondroplasia is characterized by inhibited endochondral bone growth, which **affects >90% of bones in the body** and can impact different aspects of physical development.^{3,7,9-20}

Achondroplasia is associated with potential multisystemic skeletal complications²⁰

Many complications are **more common in the first 2 years of life^{3,19-21}**



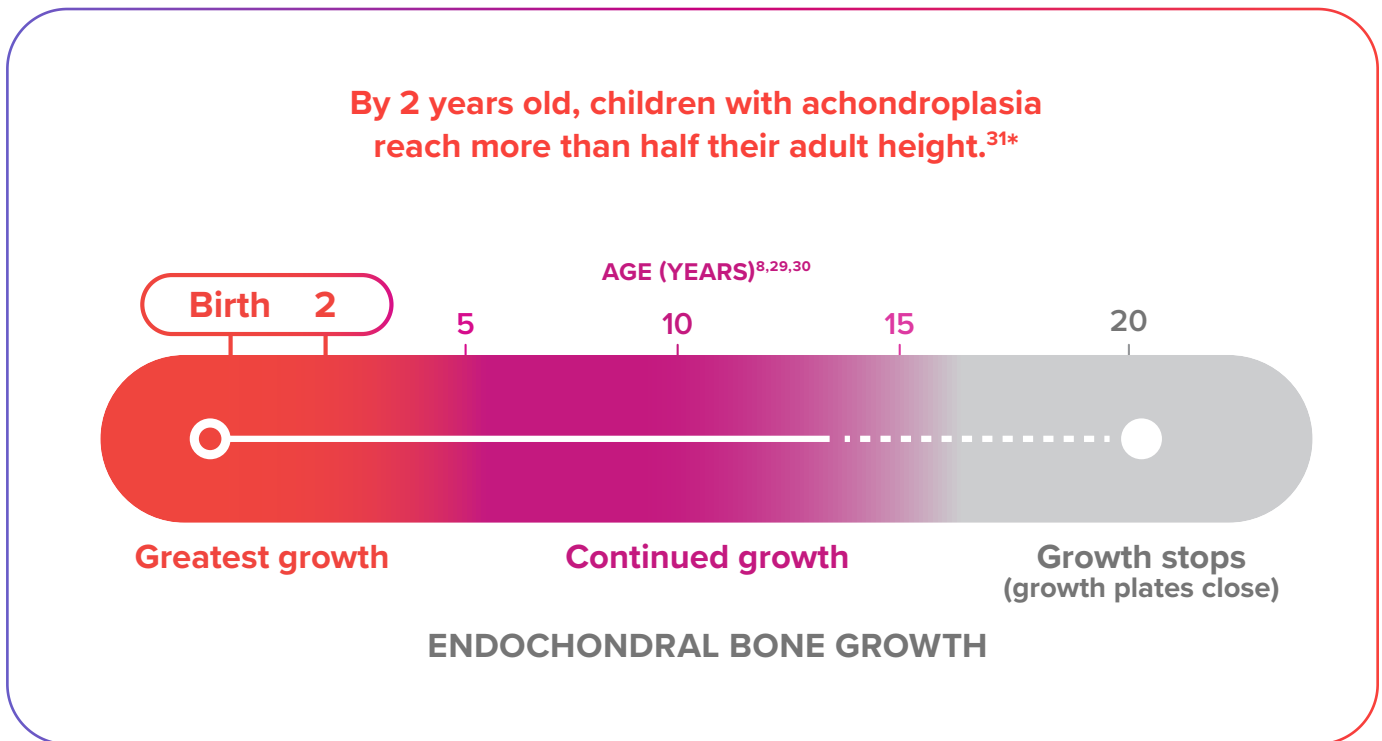
This simplified image is for illustrative purposes only.

VOXZOGO has been approved to increase linear growth in children with achondroplasia.¹ **Proactive and multidisciplinary care can help inform management and treatment approaches, which are important for multisystemic complications.^{2,3}**

Please see additional Important Safety Information presented throughout, and in the full [Prescribing Information](#).

The most rapid growth occurs in the first 2 years of life^{28,29}

During **endochondral bone growth**, cartilage is replaced by bone at open growth plates, with most bones starting to grow before birth and continuing until growth plates close.^{8,30}



In achondroplasia, an imbalance of cell signaling in growth plates inhibits endochondral bone growth throughout the body^{3,7,9,32}

*Based on stature-for-age data (birth to 18 years old) from CLARITY, an achondroplasia natural history study, comprising measurements from 549 males and 502 females with achondroplasia.³¹

IMPORTANT SAFETY INFORMATION

Adverse Reactions:

Adverse reactions that occurred in $\geq 5\%$ of patients treated with VOXZOGO and at a rate greater than that of placebo in the phase 3 study are injection site reactions (including erythema, swelling, urticaria, pain, bruising, pruritus, hemorrhage, discoloration, and induration), vomiting, arthralgia, decrease in blood pressure, gastroenteritis, diarrhea, dizziness, ear pain, influenza, fatigue, seasonal allergy, and dry skin. VOXZOGO-treated patients had an increase in alkaline phosphatase levels (17%), and was noted as a laboratory abnormality.

VOXZOGO is the first and only treatment FDA approved from birth for children with achondroplasia and open growth plates¹

By improving the balance of cell signaling, VOXZOGO targets the cause of inhibited endochondral bone growth in achondroplasia to increase linear growth—**only while growth plates remain open.**¹

Sophie

9 MONTHS OLD

on VOXZOGO since
3 months old

Efficacy and safety have been carefully evaluated over time in children with achondroplasia, including infants.^{1,33,34}

Please see additional Important Safety Information presented throughout, and in the full [Prescribing Information](#).

VOXZOGO[®]
(vosoritide) for injection

[Over 5,000] families of infants and children with achondroplasia have chosen VOXZOGO worldwide^{1,35}

Annika



Annika, 6 years old



Annika, 14 years old
On VOXZOGO since 9 years old

For illustrative purposes only, image scale is not matched.

IMPORTANT SAFETY INFORMATION

Adverse Reactions: (cont'd)

Injection site reactions: In Study 1, injection site reactions occurred in 51 (85%) subjects receiving VOXZOGO and 50 (82%) subjects receiving placebo over a 52-week period of treatment. Patients receiving VOXZOGO experienced a total of 6983 events of injection site reactions, while patients receiving placebo experienced a total of 1776 events of injection site reactions, over a 52-week period, representing 120.4 events per patient/year exposure and 29.2 events per patient/year exposure, respectively. Two patients in the VOXZOGO arm discontinued treatment due to adverse events of pain and anxiety with injections.



Ahmin

17 YEARS OLD
Completed treatment
with VOXZOGO

Resources to help guide families



VOXZOGO Mentor Program

Connect your patient's family with another caregiver who understands how they're feeling.



VOXZOGO Stories

Meet families who chose VOXZOGO and gain insights from their advice.



Achondroplasia Doctor Finder

Refer your patients' families to a network of specialized care in their area.

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Warnings and Precautions for Risk of Low Blood Pressure

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Pediatric Patients 0 to <5 Years:

The safety of VOXZOGO in pediatric patients 0 to <5 years with achondroplasia was evaluated in a 52-week randomized, double-blind, placebo-controlled study (Study 2). In this study, 64 patients from birth to <5 years of age were randomized to receive either a daily vosoritide dose with similar exposure to that characterized to be safe and effective in children with ACH aged ≥ 5 years old, or placebo. An additional 11 patients received open-label treatment as part of this study. The most common adverse reactions ($>10\%$) reported in pediatric patients 0 to <5 years were injection site reactions (86%) and rash (28%). The overall safety profile of VOXZOGO in pediatric patients 0 to <5 years was similar to that seen in older pediatric patients.

Administration and Monitoring:

VOXZOGO is administered as a daily subcutaneous injection. Prior to use, instruct caregivers on proper preparation and administration of VOXZOGO, and ensure caregivers have demonstrated the ability to perform a subcutaneous injection.

Monitor and assess patient body weight, growth, and physical development regularly every 3-6 months. Adjust dosage according to the patient's actual body weight. Permanently discontinue treatment with VOXZOGO upon confirmation of no further growth potential, indicated by closure of epiphyses.

Special Populations:

- There are no available data on the use of VOXZOGO in pregnant women, or data on the presence of VOXZOGO in human milk, the effects on the breastfed infant, or the effects on milk production.
- The influence of renal impairment on the pharmacokinetics of VOXZOGO has not been evaluated. No dosage adjustment is needed for patients with $eGFR \geq 60$ mL/min/1.73 m². VOXZOGO is not recommended for patients with $eGFR < 60$ mL/min/1.73 m².

You may report side effects to the FDA at **1-800-FDA-1088** or **www.fda.gov/medwatch**. You may also report side effects to BioMarin at **1-866-906-6100**.

Please see additional safety information in the full [Prescribing Information](#).



Elijah

**20 MONTHS OLD
on VOXZOGO since
12 months old**

References: 1. VOXZOGO [package insert]. Novato, CA: BioMarin Pharmaceutical Inc; 2024. 2. Hoover-Fong J, Scott CI, Jones MC; Committee on Genetics. Health supervision for people with achondroplasia. *Pediatrics*. 2020;145(6):e20201010. 3. Savariayan R, Ireland P, Irving M, et al. International Consensus Statement on the diagnosis, multidisciplinary management and lifelong care of individuals with achondroplasia. *Nat Rev Endocrinol*. 2022;18(3):173-189. 4. Hoover-Fong JE, Alade AY, Hashmi SS, et al. Achondroplasia Natural History Study (CLARITY): a multicenter retrospective cohort study of achondroplasia in the United States. *Genet Med*. 2021;23(8):1498-1505. 5. Cormier-Daire V, AlSayed M, Alves I, et al. Optimising the diagnosis and referral of achondroplasia in Europe: European Achondroplasia Forum best practice recommendations. *Orphanet J Rare Dis*. 2022;17(1):293. 6. Savariayan R, Hoover-Fong J, Ozono K, et al. 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Savariayan R, Wilcox WR, Harmatz P, et al. Persistence of growth-promoting effects in infants and toddlers with achondroplasia: results from a Phase 2 extension study with vosoritide. Annual Clinical Genetics Meeting (ACMG), Toronto, Canada. 2024. Poster. P131. 35. Data on file [1]. BioMarin Pharmaceutical Inc; 2025.

Early intervention starts with you^{2,3}

Only VOXZOGO is FDA approved from birth to increase linear growth in infants and children with achondroplasia while growth plates remain open.¹ Help your patients maximize time on treatment with a timely referral to specialized care.^{1-3,6}



Evelyn

20 MONTHS OLD
on VOXZOGO since
12 months old as part
of a clinical trial

VOXZOGO[®]
(vosoritide) for injection



Connect with a BioMarin representative

Dedicated account managers can help answer questions about treatment with VOXZOGO and outline next steps for getting your patients started.¹

IMPORTANT SAFETY INFORMATION

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