



Evelyn

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

Approaching care with confidence

A guide to talking with doctors about care for your child with achondroplasia, including a treatment option available as early as birth

VOXZOGO is the only treatment FDA approved from birth to increase linear growth for children with achondroplasia and open growth plates.

VOXZOGO[®]
(vosoritide) for injection

IMPORTANT SAFETY INFORMATION

What is the most important safety information about VOXZOGO?

- VOXZOGO may cause serious side effects including a temporary decrease in blood pressure in some patients. To reduce the risk of a decrease in blood pressure and associated symptoms (dizziness, feeling tired, or nausea), patients should eat a meal and drink 8 to 10 ounces of fluid within 1 hour before receiving VOXZOGO.

Please see Important Safety Information throughout, as well as the included full [Prescribing Information](#) and [Patient Information](#).

Navigating a diagnosis may be overwhelming at first

Achondroplasia is a rare condition that inhibits bone growth throughout the body, with the **greatest impact on growth occurring in the first 2 years**. This means that children with achondroplasia experience the greatest potential for growth early in life.

Because inhibited bone growth can affect multiple aspects of physical development, your child may need to see different doctors over time to help address their unique and changing needs. As you navigate these appointments and decisions, remember that your insight into your child's needs and personality is invaluable—it helps guide care that's truly centered on them.

At the start of this healthcare journey, it may be challenging to know where to begin and which questions to ask. **But you know your child best, and you are their greatest advocate.**



Use these resources to help guide you and have more informed discussions with your child's doctors.

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Corinna

20 MONTHS OLD

on VOXZOGO since 8 months old as part of a clinical trial

“ This journey and making the decisions we’ve had to make, it’s really brought our family closer together. ”

- Sarah, Corinna’s mom

IMPORTANT SAFETY INFORMATION

What is VOXZOGO used for?

- VOXZOGO is a prescription medicine used to increase linear growth in children with achondroplasia and open growth plates (epiphyses).
- VOXZOGO is approved under accelerated approval based on an improvement in annualized growth velocity. Continued approval may be contingent upon verification and description of clinical benefit in confirmatory trials.

Exploring expert-created resources

Because achondroplasia is rare, you and your child's doctors may need additional information to guide your discussions. Below are two achondroplasia care resources created by expert medical professionals and patient advocates that help to provide standardized guidance from birth and throughout development.



2020 AAP Achondroplasia Management Guidelines

A clinical report from the American Academy of Pediatrics (AAP) on potential health and developmental impacts, what can be done based on your child's age, and the importance of lifelong comprehensive care.

Key callouts based on your child's age:

- Infants may require extra attention and supervision to monitor for potential impacts on breathing or the nervous system
- Children should continue regular checkups to ensure milestones are being met and to monitor for potential health effects
- Teens and adults may require ongoing care with a focus on mental health and mobility



2025 International Consensus Treatment Guidelines

A list of evidence-based recommendations created by a global team of healthcare professionals, and two patient advocates, on how to navigate treatment with vosoritide (VOXZOGO).

Key recommendations include:

- Receive educational information on VOXZOGO from your care team
- Begin treatment as early as possible
- Discuss personalized treatment goals for your child

BioMarin provided funding for the *International consensus guidelines on the implementation and monitoring of vosoritide therapy in individuals with achondroplasia*, including payment to participants. BioMarin was not involved in deciding the guidelines content, including who were involved in developing the guidelines. For details, please see the Acknowledgements section of the publication.

Getting the most out of treatment discussions

Your child's appointments may feel rushed or overwhelming at times. Reference these resources and questions to take full advantage of every appointment.

Questions to consider:

- Can you tell me about your experience caring for children with achondroplasia?
- Can I share and discuss resources with you to help deepen your knowledge of achondroplasia?
- Is there anything we should be watching out for at home?
- Are there other healthcare providers my child may need to see?
- What can you tell me about the ability to begin VOXZOGO from birth?
- How can we make the most of our experience with VOXZOGO?

It's important to understand all your options so you can choose what's best for your family.

IMPORTANT SAFETY INFORMATION

What are the most common side effects of VOXZOGO?

- The most common side effects of VOXZOGO include injection site reactions (including redness, itching, swelling, bruising, rash, hives, and injection site pain), high levels of blood alkaline phosphatase shown in blood tests, vomiting, joint pain, decreased blood pressure, and stomach ache. These are not all the possible side effects of VOXZOGO. Ask your healthcare provider for medical advice about side effects, and about any side effects that bother the patient or that do not go away.

Expanding your network and resources

Find guidance from experienced healthcare professionals and hear from the families who chose VOXZOGO through these additional resources.



Achondroplasia Doctor Finder

Find healthcare providers in your area who are experienced with caring for children with achondroplasia.



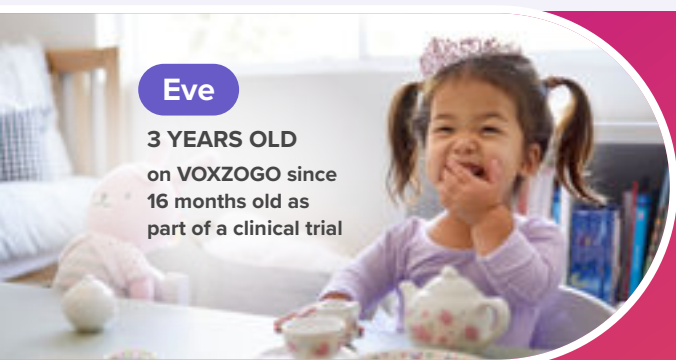
VOXZOGO Caregiver Brochure

Learn why more and more families are choosing treatment with VOXZOGO from as early as birth.



VOXZOGO Patient Stories

Hear from the families who have experienced the impact of VOXZOGO and see if it's the right choice for your child.



Eve

3 YEARS OLD
on VOXZOGO since
16 months old as
part of a clinical trial

“There was a limited timeframe on how much growth she has. That’s what made our decision to start treatment early.”

- Julie, Eve’s mom

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Important Safety Information

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How is VOXZOGO taken?

- VOXZOGO is taken daily as an injection given under the skin, administered by a caregiver after a healthcare provider determines the caregiver is able to administer VOXZOGO. Do not try to inject VOXZOGO until you have been shown the right way by your healthcare provider. VOXZOGO is supplied with Instructions for Use that describe the steps for preparing, injecting, and disposing VOXZOGO. Caregivers should review the Instructions for Use for guidance and any time they receive a refill of VOXZOGO in case any changes have been made.

- Inject VOXZOGO 1 time every day, at about the same time each day. If a dose of VOXZOGO is missed, it can be given within 12 hours from the missed dose. After 12 hours, skip the missed dose and administer the next daily dose as usual.
- The dose of VOXZOGO is based on body weight. Your healthcare provider will adjust the dose based on changes in weight following regular check-ups.
- Your healthcare provider will monitor the patient's growth and tell you when to stop taking VOXZOGO if they determine the patient is no longer able to grow. Stop administering VOXZOGO if instructed by your healthcare provider.

What should you tell the doctor before or during taking VOXZOGO?

- Tell your doctor about all of the patient's medical conditions including
 - If the patient has heart disease (cardiac or vascular disease), or if the patient is on blood pressure medicine (anti-hypertensive medicine).
 - If the patient has kidney problems or renal impairment.
 - If the patient is pregnant or plans to become pregnant. It is not known if VOXZOGO will harm the unborn baby.
 - If the patient is breastfeeding or plans to breastfeed. It is not known if VOXZOGO passes into breast milk.
- Tell your doctor about all of the medicines the patient takes, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

You may report side effects to BioMarin at **1-866-906-6100**. You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call **1-800-FDA-1088**.

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

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Vihaan

7 YEARS OLD

on VOXZOGO since
5½ years old as part
of a clinical trial

Connect with an experienced caregiver



The VOXZOGO Mentor Program

Meet with a caregiver raising a child on VOXZOGO to ask questions, find guidance, and gain valuable insight from families like yours.

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