



Here for you from the start

With 10+ years of clinical trial
experience in achondroplasia

Vivan

18 YEARS OLD

on VOXZOGO for ~10 years
until growth plates closed,
as part of a clinical trial

VOXZOGO[®]
(vosoritide) for injection

The only treatment FDA approved
for children with achondroplasia
from birth until growth plates close.

INDICATION AND IMPORTANT SAFETY INFORMATION

- VOXZOGO[®] (vosoritide) 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.

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 full [Prescribing Information](#) and [Patient Information](#).

More and more families are choosing VOXZOGO

Join the over 4,000 families around the world who decided that treatment with VOXZOGO was the right choice for their infants and children with achondroplasia.

Together, they are discovering the impact treatment can make—and are starting as early as birth.

VOXZOGO[®]
(vosoritide) for injection

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.



Vihaan

7 YEARS OLD

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

**“I heard lots of good stories about VOXZOGO.
If it can help my son, I’m going to try that.”**

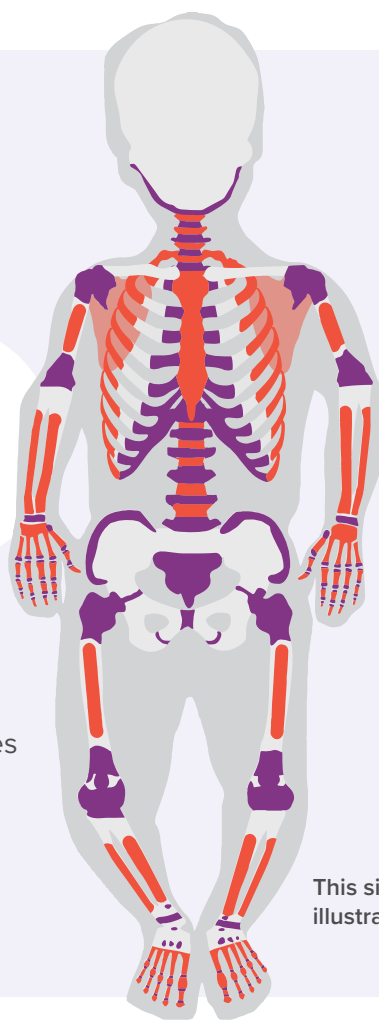
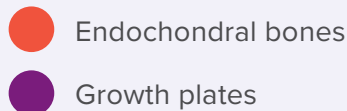
- Ridhi, Vihaan’s mom

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The impact of inhibited bone growth

In achondroplasia, the growth of bones that develop from cartilage (called endochondral bones) is inhibited. This **can impact physical development throughout the body**, including reduced and disproportionate growth.

Endochondral bone growth is inhibited in achondroplasia—that's over 90% of the body's bones



This simplified image is for illustrative purposes only.

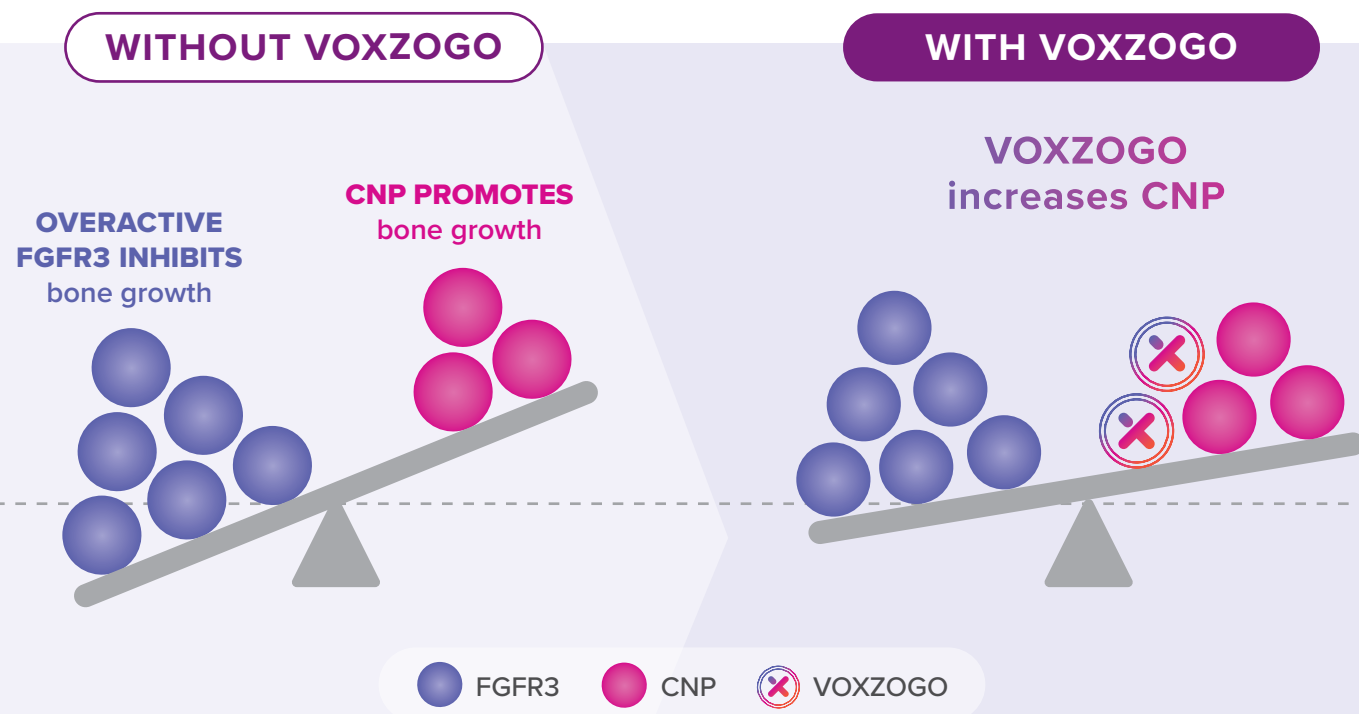
IMPORTANT SAFETY INFORMATION

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.

Bone growth is inhibited by imbalanced cell signals

Bone growth requires a balance of two cell signals—CNP (which promotes growth) and FGFR3 (which slows growth). In achondroplasia, the FGFR3 signals are overactive, which inhibits bone growth. **VOXZOGO** targets the cause of inhibited bone growth.



This graphic is an illustration of the cell signaling imbalance in achondroplasia and how VOXZOGO is designed to improve the balance. This graphic is not to scale and does not reflect a percentage growth.

VOXZOGO helps to improve the balance and promote bone growth.

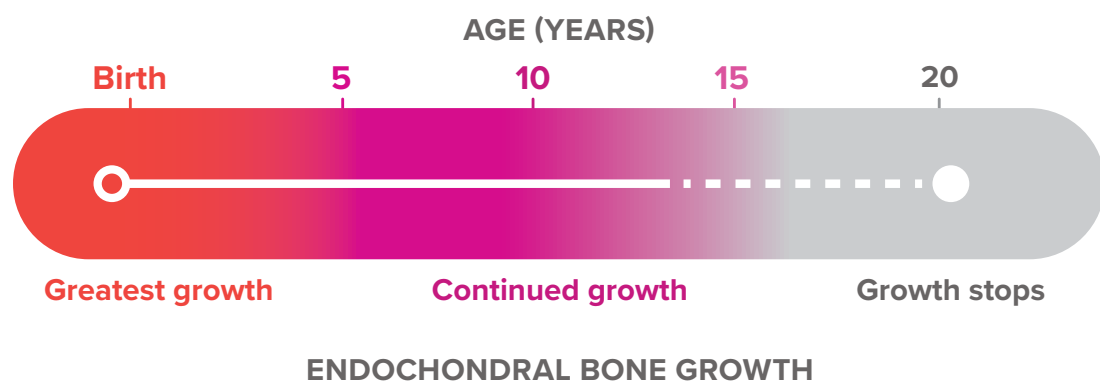
CNP, C-type natriuretic peptide; **FGFR3**, fibroblast growth factor receptor 3.

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VOXZOGO[®]
(vosoritide) for injection

VOXZOGO supports bone growth as early as birth

Bones only grow for a limited time—with the most rapid growth occurring in the first 2 years of life



The only treatment FDA approved from birth to increase linear growth* for infants and children with achondroplasia while they are still growing (before growth plates have closed)

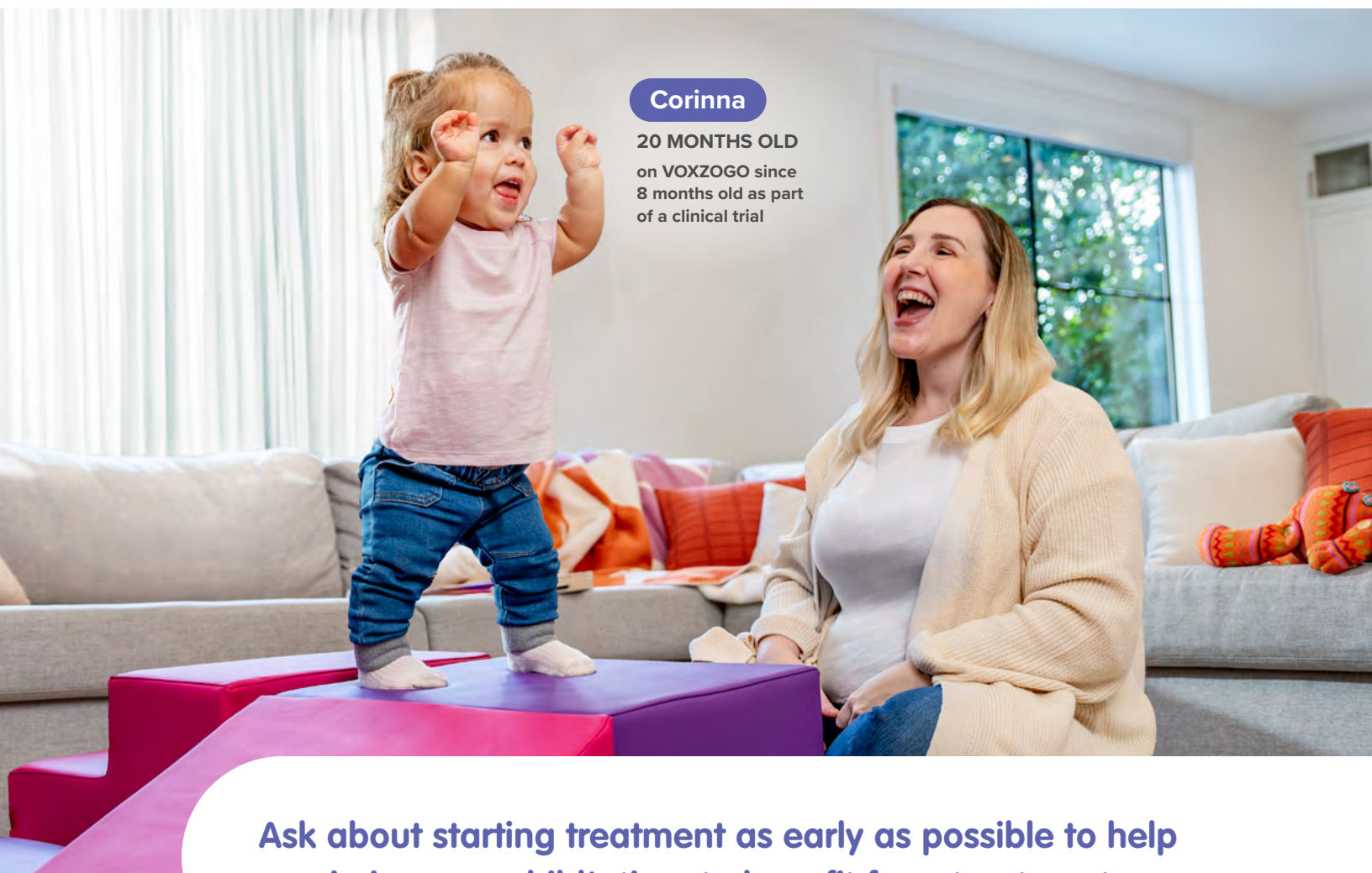
*Linear growth, an increase in height, is an indicator of overall health and development.

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VOXZOGO[®]
(vosoritide) for injection

Medical guidelines support starting VOXZOGO early and staying on treatment until bones stop growing

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.



Corinna

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

Ask about starting treatment as early as possible to help maximize your child's time to benefit from treatment.

IMPORTANT SAFETY INFORMATION

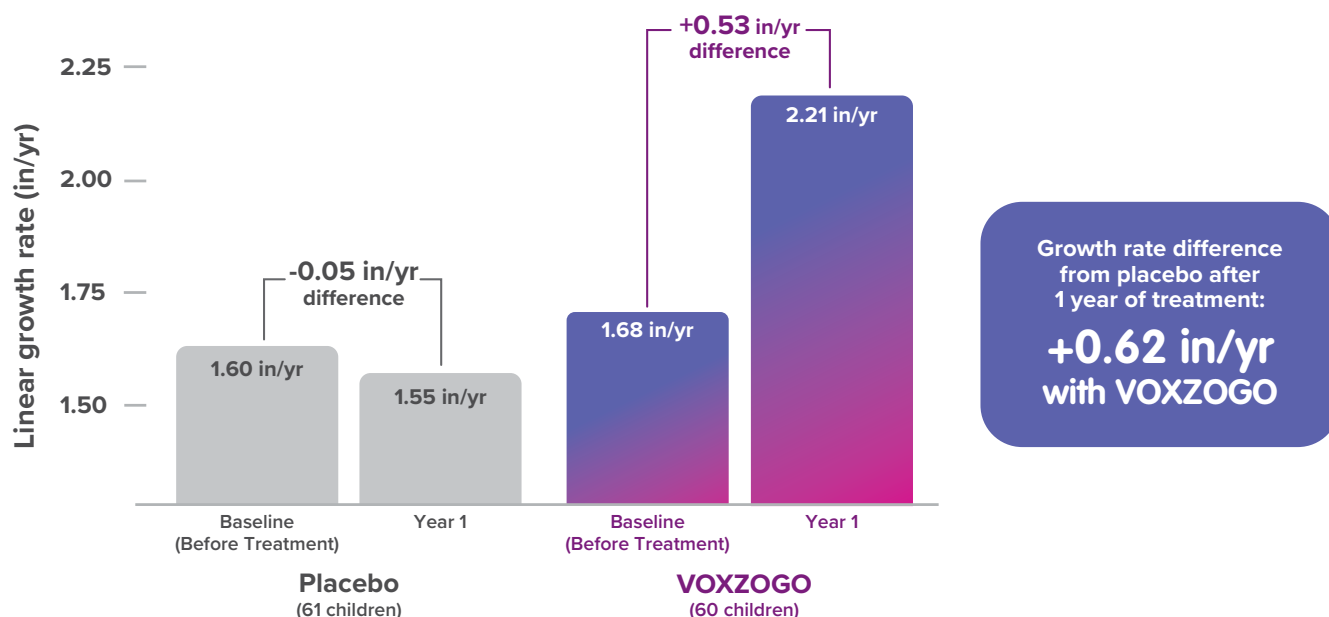
How is VOXZOGO taken? (cont'd)

- 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.

Children with achondroplasia can grow faster on VOXZOGO than without treatment

In a clinical trial of children aged 5 to 15 years, the impact of VOXZOGO on linear growth was measured over 1 year.

Growth rate before and after 1 year of treatment



**Improvement in linear growth over baseline
(from when treatment was started)
was maintained over 4 years in an ongoing study.**

At year 4, the rate of growth was 1.88 in/yr.*

97% of children (58/60) stayed on VOXZOGO during the first year.†

*These results are from the open-label extension study, which did not have a placebo group, and caregivers, patients, and doctors were aware that all children were given VOXZOGO.

†One child discontinued due to injection pain, and one child discontinued due to fear of needles.

IMPORTANT SAFETY INFORMATION

How is VOXZOGO taken? (cont'd)

- 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.

The impact of VOXZOGO continues to be studied

VOXZOGO is approved under accelerated approval based on an improvement in growth rate after 1 year of treatment. Compared to untreated patients in the study, body proportionality did not worsen at 1 year.

Because achondroplasia is a condition that affects multiple aspects of physical development, additional studies on the impact of VOXZOGO over a longer period of time are ongoing, including its effects on final adult height and body proportionality.



Zeke

7 YEARS OLD
on VOXZOGO since
4½ years old as part
of a clinical trial



Learn more about the impact of VOXZOGO

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

VOXZOGO
(vosoritide) for injection

Meet the community

Get to know families like yours who have chosen VOXZOGO and witnessed the impact that increased linear growth can make.



Eve

3 YEARS OLD

Time on VOXZOGO: 1½ years

Eve is fierce, smart, and very independent. Whether it's starting ballet or beginning treatment, her parents wanted to make every effort to support her. They made the decision to start VOXZOGO early because they knew there was a limited time for growth.

"We were able to do something very actionable for Eve, give her the benefit of potential growth. We try to share those benefits with other parents."

- Julie, Eve's mother

IMPORTANT SAFETY INFORMATION

How is VOXZOGO taken? (cont'd)

- 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.



Vivan

18 YEARS OLD

Time on VOXZOGO: ~10 years, completed treatment

Vivan isn't shy when it comes to the challenges he's faced, but he hasn't let that keep him from dreaming big. From playing basketball to completing treatment, he sets goals and stays committed to seeing them through.

"If I like something, I'm probably going to try to commit to that as much as possible."

- Vivan



See why more families chose VOXZOGO

Meet other families who understand the importance of starting early and staying on treatment until bones stop growing.

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VOXZOGO
(vosoritide) for injection

Understanding the safety of VOXZOGO over time

Children 5 to 15 years old

Below are the most common side effects seen in over 10% of children (aged 5 to 15 years) on VOXZOGO over 1 year in a clinical trial.*

SIDE EFFECT	PLACEBO (n=61)	VOXZOGO (n=60)
Injection site reddening (erythema) [†]	42 (69%)	45 (75%)
Injection site swelling [†]	22 (36%)	37 (62%)
Vomiting	12 (20%)	16 (27%)
Injection site hives (urticaria) [†]	6 (10%)	15 (25%)
Joint pain (arthralgia)	4 (7%)	9 (15%)
Decreased blood pressure	3 (5%)	8 (13%)
Stomach ache (gastroenteritis) [‡]	5 (8%)	8 (13%)

In ongoing studies of VOXZOGO in these children, the side effects observed for up to 6 years of treatment are similar to those seen in the first year.



It's important to know:

- VOXZOGO may cause serious side effects, including a temporary decrease in blood pressure in some patients
- You can reduce the risk of decreased blood pressure and associated symptoms (dizziness, feeling tired, or nausea) by following these steps in the hour before receiving VOXZOGO:
 - Be well fed
 - Be well hydrated

*Includes adverse reactions occurring in over 10% of VOXZOGO patients and more frequently in the VOXZOGO arm with a risk difference of $\geq 5\%$ (ie, difference of >2 subjects) between treatment arms.

[†]Injection site reactions occurring more frequently in VOXZOGO-treated subjects than placebo.

[‡]Includes the preferred terms: gastroenteritis and gastroenteritis, viral.



Evelyn

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

Children under 5 years old

The safety of VOXZOGO in children under 5 years old was similar to that seen in children ages 5 years and up.

Over 1 year, the most common side effects seen in over 10% of children on VOXZOGO (under 5 years old) were injection site reactions (86%) and rash (28%).

In ongoing studies of VOXZOGO in these children, the side effects observed for up to 4 years of treatment are similar to those seen in the first year.

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VOXZOGO
(vosoritide) for injection

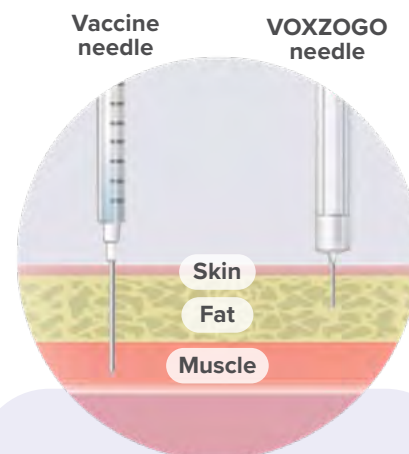
A routine that can fit into your daily life

VOXZOGO is an at-home daily injection that's given with a short, thin needle—smaller than the one used for most vaccinations.



Eve

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



While routine vaccinations, like a flu shot, go into the muscle, **VOXZOGO is given just under your child's skin**

This image is not intended to show injection technique. Always refer to the **Instructions for Use** when giving **VOXZOGO** to your child.

Your child's doctor will train you on how to give **VOXZOGO and your BioMarin Clinical Coordinator will be available to reinforce training.**

IMPORTANT SAFETY INFORMATION

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.

Clinical Coordinators are here to help

One-to-one support for you and your family



- Providing product education and support around the injection process
- Helping to find an injection routine that works for your family throughout your child's entire time on treatment
- Assisting through the specialty pharmacy process and sharing reminders for product refills
- Offering flexible meeting options including in person and/or virtually, by email, phone, or text



Shawn

8 YEARS OLD

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

**"Families know that they can call or text us
anytime if they have any questions or concerns."**

- Christina, BioMarin Clinical Coordinator

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VOXZOGO
(vosoritide) for injection

Maintaining your routine

Build confidence with your child to help start and continue on their routine. Establish your reason for choosing VOXZOGO to keep you and your family on course for the duration of treatment.



Identify goals and appropriate expectations

Talk about what you and your family hope to get out of treatment—set goals and revisit them over time as your child progresses.

Every child is unique and may respond to treatment differently.
Work with your doctor to set realistic expectations for your child.



Lean on your Clinical Coordinator

You will be paired with a **BioMarin Clinical Coordinator** who will provide you with ongoing product education and support throughout the duration of your child's treatment.



Track progress

It's typical to see results after 1-2 years of starting treatment,* making regular growth and weight checkups an important part of your routine.

Children's growth rates naturally vary over time. While on VOXZOGO, your child's growth rate may slow down or stay the same over a period of time, but this does not mean it is not working. Stay the course by celebrating every milestone and reflecting on why you decided to start treatment.

*This is a select recommendation from the *International consensus guidelines on the implementation and monitoring of vosoritide therapy in individuals with achondroplasia*.

IMPORTANT SAFETY INFORMATION

What should you tell the doctor before or during taking VOXZOGO? (cont'd)

- 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**.



Elijah

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

**“Try it, do it, and
stay consistent.”**

- Marvin, Elijah’s dad



Meet with your doctor regularly

Your child’s frequent appointments are a great opportunity to ask questions and have open, productive conversations about treatment.

Work with your child’s doctor to:

- Get measurements taken every 3 to 6 months
- Get growth-specific monitoring from a specialist, such as a pediatric endocrinologist or geneticist



Find experienced care for your child

Scan the QR code to connect with local providers who have chosen to be part of the **Achondroplasia Doctor Finder**.



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VOXZOGO
(vosoritide) for injection

With you every step of the way

Personalized assistance for your family



BioMarin RareConnections™

Case and Field Reimbursement Managers help navigate coverage by:

- Helping with the insurance process and sharing coverage options
- Identifying financial programs, such as co-pay assistance, if you're an eligible commercially insured patient*
- Offering guidance on insurance changes
- Ensuring the specialty pharmacy receives your prescription
- Educating your doctor on coverage requirements for continued access to therapy

Widespread coverage to help you get started



**OVER
96%**

**of insured patients have secured
coverage for VOXZOGO[†]**



**OVER
96%**

**of eligible families with commercial insurance
paid \$0 out-of-pocket for VOXZOGO^{*†}**

*With the BioMarin Co-Pay Assistance Program. Terms and Conditions apply. Valid only for patients with commercial prescription insurance coverage who have a valid prescription for an FDA-approved indication and who meet additional eligibility criteria. Not valid for prescriptions reimbursed, in whole or in part, by any federal, state, or government-funded insurance programs (for example, Medicare, Medicare Advantage, Medigap, Medicaid, VA, DoD, or TRICARE) or where prohibited by law or by the patient's health insurance provider. If at any time a patient begins receiving prescription drug coverage under any federal, state, or government-funded healthcare program, the patient will no longer be able to use the program and patient must notify BioMarin RareConnections at 1-866-906-6100 to stop participation. Patients residing in or receiving treatment in certain states may not be eligible for some or all of the program elements. Patients may not seek reimbursement for value received from the program from any third-party payers. Additional restrictions may apply. Offer subject to change or discontinuance without notice. This assistance offer is not health insurance. See [BioMarin-copay-terms.com](https://biomarin-copay-terms.com) for full Terms and Conditions.

[†]BioMarin RareConnections™ Data on file. Patients included are eligible VOXZOGO patients who have enrolled in BioMarin RareConnections and are on commercial therapy.




Cal

12 YEARS OLD
on VOXZOGO since
10½ years old as
part of a clinical trial

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**"My advice to parents, trust yourselves.
Trust your instincts and know that
you have the ability to figure it out
and can find the answers you need."**

- Krista, Annika's mom

Annika

14 YEARS OLD
on VOXZOGO since
9 years old as part
of a clinical trial

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VOXZOGO[®]
(vosoritide) for injection

Find connection, support, and advice within the achondroplasia community

Little People of America

Little People of America (LPA) is the largest and longest-standing organization supporting people with dwarfism. With a national network of chapters, LPA provides peer support, education, and advocacy for individuals and families. The organization champions disability rights while celebrating the identities and lived experiences of people with short stature.

lpaonline.org



Growing Stronger

Growing Stronger's mission is to improve the quality of medical care for little people through supporting research.

growingstronger.org



The Chandler Project

The Chandler Project brings together the global achondroplasia community and spreads awareness to its network of patients, parents, and caregivers seeking information on the latest treatment and medical research in achondroplasia.

thechandlerproject.org



Little Legs Big Heart

The Little Legs Big Heart Foundation is on a mission to empower and uplift individuals living with skeletal dysplasia and their families.

littlelegsbigheartfoundation.org



Your network may be larger than you think.

Important Safety Information

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What is the most important safety information about VOXZOGO?

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What are the most common side effects of VOXZOGO?

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- 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](#).

A trusted leader in achondroplasia



4,000+ infants and children with achondroplasia
have been prescribed VOXZOGO worldwide



**The longest studied treatment for achondroplasia
with 10+ years of clinical trial experience**



**Personalized support with you every
step of the way**

**Talk to your doctor about starting VOXZOGO early
and staying on until growth plates close.**

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

Join the thousands of families worldwide who have chosen VOXZOGO

VOXZOGO[®]
(vosoritide) for injection

The only treatment FDA approved from birth until growth plates close to target the underlying cause of inhibited bone growth for children with achondroplasia



Miller

13 YEARS OLD
on VOXZOGO since
10 years old as part
of a clinical trial

**Connect with
an experienced
caregiver**



The VOXZOGO Mentor Program pairs you with a caregiver raising a child on VOXZOGO. Ask questions, find guidance, and gain valuable insights from someone who's been there before.

IMPORTANT SAFETY INFORMATION

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B:OMARIN[®]