

# Approved from birth. Backed by experience.

5,000+ infants and children with achondroplasia  
have been prescribed VOXZOGO worldwide<sup>1-3</sup>

Elijah

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



**VOXZOGO**<sup>®</sup>  
(vosoritide) for injection

**The only treatment FDA approved from  
birth until growth plates close to increase  
linear growth for children with achondroplasia.<sup>1</sup>**

## **INDICATION AND IMPORTANT SAFETY INFORMATION**

**VOXZOGO**<sup>®</sup> (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 full [Prescribing Information](#).

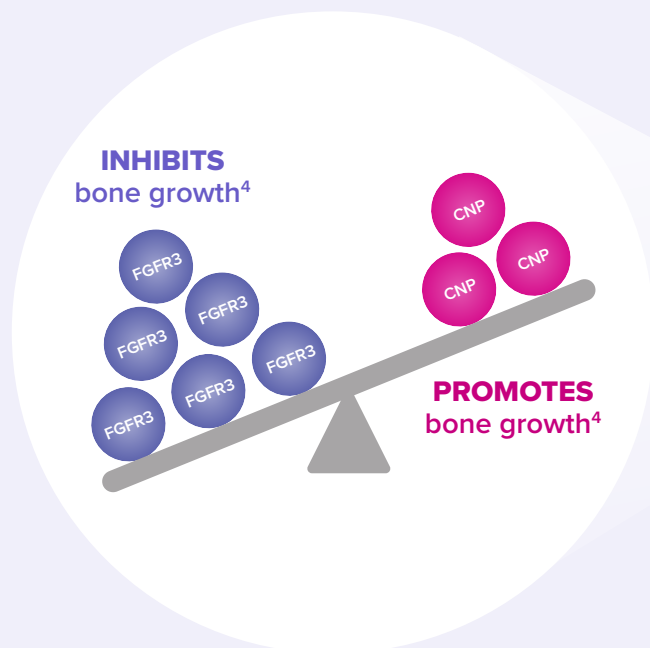
# Endochondral bone growth is inhibited in achondroplasia<sup>4,5</sup>

## Endochondral bone growth

is the predominant process of bone development in which cartilage is replaced by bone at open growth plates.<sup>6,7</sup>

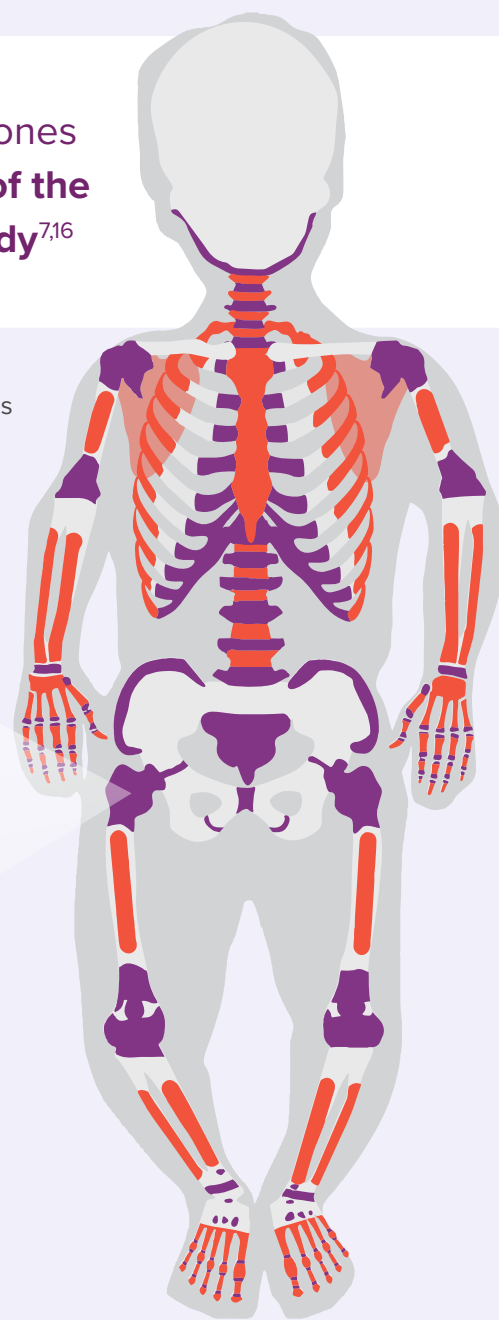
Endochondral bones make up **>90% of the bones in the body**<sup>7,16</sup>

-  Endochondral bones
-  Growth plates<sup>6,9,17-25</sup>



**Overactive FGFR3 signaling relative to CNP signaling** in growth plate cells is the underlying cause of inhibited bone growth in achondroplasia<sup>4</sup>

Endogenous CNP levels cannot adequately regulate overactive FGFR3 signaling.<sup>4</sup>



This simplified image is for illustrative purposes only.

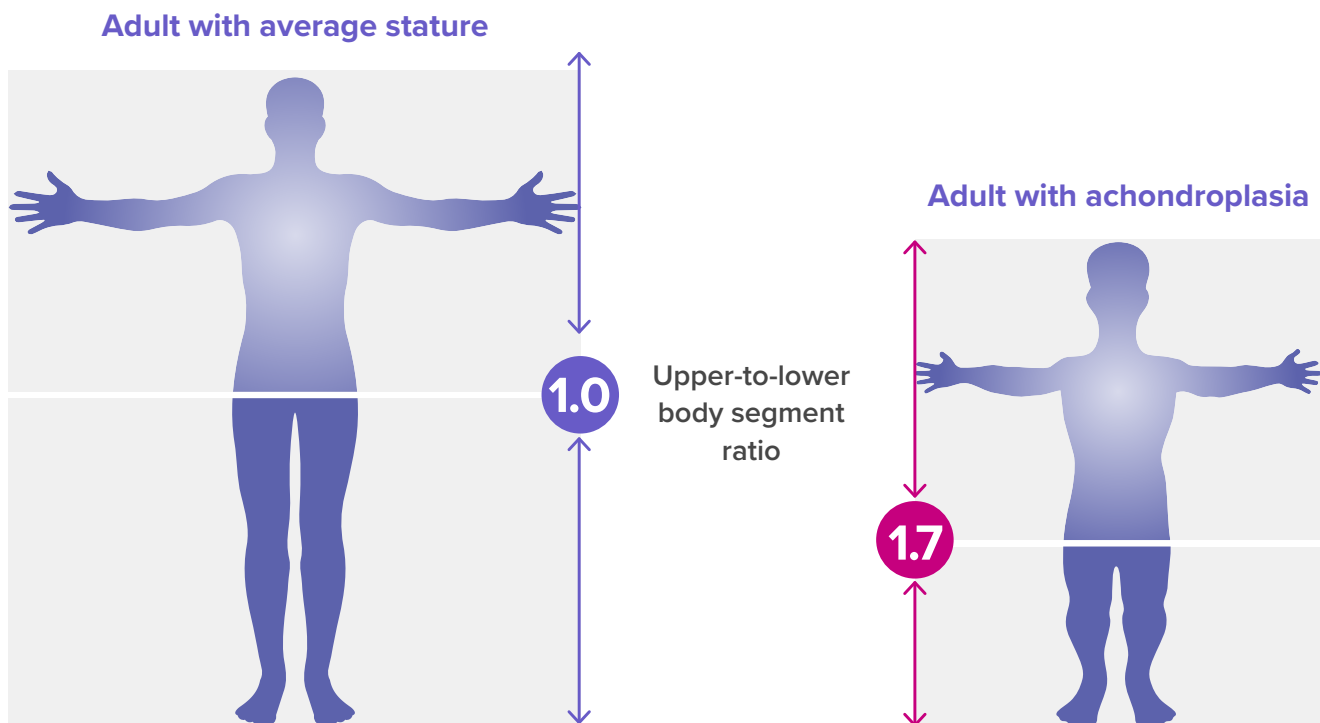
**CNP**, C-type natriuretic peptide; **FGFR3**, fibroblast growth factor receptor 3.

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

# Inhibited bone growth throughout the body can affect different aspects of development<sup>4-7,26,27</sup>

Inhibited endochondral bone growth leads to distinct characteristic features such as **reduced and disproportionate growth**<sup>4,5,26</sup>

**Upper-to-lower body segment ratio** is a common measure of body proportionality and is calculated by the length of the upper body divided by the length of the lower body.<sup>28-30</sup>



Upper-to-lower body segment ratio becomes proportional at 10 years old (ratio=1).<sup>28,29</sup>

Upper-to-lower body disproportionality continues into adulthood (ratio>1).<sup>28,29</sup>

## IMPORTANT SAFETY INFORMATION

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

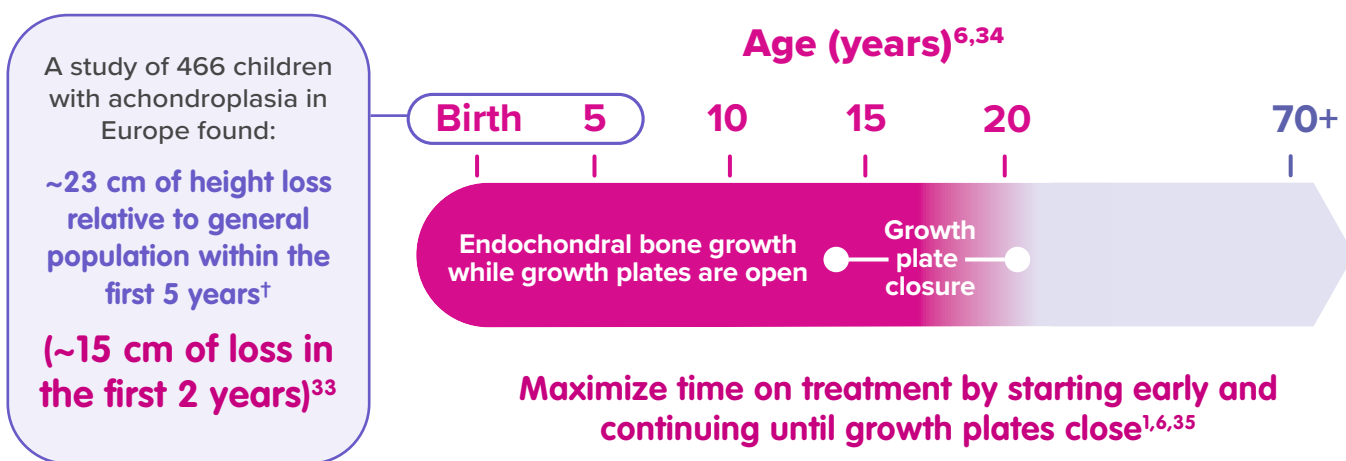
In a 52-week, randomized, double-blind, placebo-controlled trial in 121 subjects with achondroplasia, subjects aged from 5.1 to 14.9 years, (Study 1) eight (13%) of 60 patients treated with VOXZOGO had a total of 11 events of transient decrease in blood pressure, compared to 3 (5%) of 61 patients on 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.

# VOXZOGO promotes endochondral bone growth as early as birth<sup>1</sup>

VOXZOGO targets overactive FGFR3 signaling and works by mimicking the body's natural CNP to promote endochondral bone growth, but only while growth plates remain open.<sup>1</sup>

## By 2 years old, children with achondroplasia reach more than half their adult height<sup>31\*</sup>

Growth deficit in achondroplasia accumulates over time<sup>4,28,32</sup>



## International consensus guidelines support early initiation of VOXZOGO<sup>35</sup>

BioMarin provided funding for the *International consensus guidelines on the implementation and monitoring of vosoritide therapy in individuals with achondroplasia*, including honoraria to participants. BioMarin was not involved in the selection of the guidelines development group, defining the guidelines scope, the voting process, analysis of the results, or preparation of the submitted manuscript. Please see Acknowledgements section of publication for additional detail.<sup>35</sup>



SCAN THE QR CODE TO LEARN MORE

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

†For boys, about 51% of total height deficit at adulthood occurred during the first 5 years of life, and 59% for girls. A mix of cross-sectional and longitudinal, retrospective, and prospective data from 466 children with achondroplasia and 4,375 measuring occasions were modeled with generalized additive model for location, scale, and shape to sex-specific references for ages 0 to 20 years.<sup>33</sup>

# VOXZOGO has been studied in children aged 4 months to <18 years<sup>1,36-38</sup>

## **CANOPY ACH-OS (Study 901)** Ongoing Observational Study<sup>37,39</sup>

- Age: 0 months to <18 years
- Establish baseline growth\*



## **CANOPY ACH-2I (Study 206)<sup>†</sup>** Phase 2 Infant and Toddler Study<sup>38,39</sup>

- Age: 0 months to <5 years
- 52-week, double-blind, randomized, placebo-controlled

## **CANOPY ACH-EXT (Study 208)** Ongoing Open-label Extension Study<sup>39,40</sup>

- Duration: Near final adult height

## **CANOPY ACH-3 (Study 301)** Phase 3 Pivotal Study<sup>36,39</sup>

- Age: 5 to <18 years
- 52-week, double-blind, randomized, placebo-controlled

## **CANOPY ACH-EXT (Study 302)** Ongoing Open-label Extension Study<sup>36,39,41</sup>

- Duration: Final adult height

**Data continue to be collected in ongoing clinical trials<sup>2</sup>**

\*Duration of ≥6 months if aged ≥3 months at study entry; duration of ≥3 months if aged <3 months at study entry.<sup>37</sup>

<sup>†</sup>Participants completed at least 3 or 6 months of an observational run-in growth study (either in CANOPY ACH-OS [Study 901] or CANOPY ACH-2I [Study 206]) to establish their baseline annualized growth velocity.<sup>38,39</sup>

## **IMPORTANT SAFETY INFORMATION**

### **Adverse Reactions:**

Adverse reactions that occurred in ≥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.

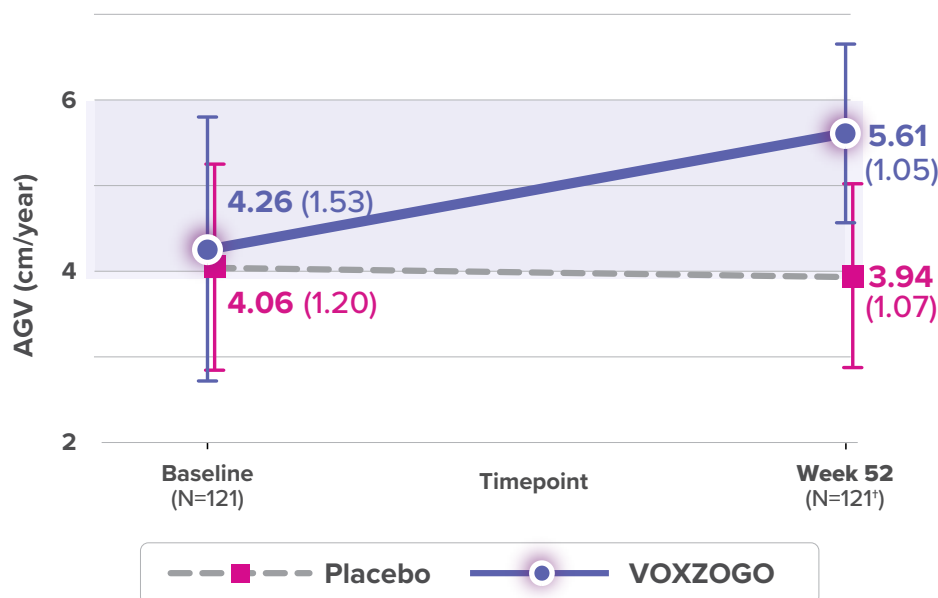
**VOXZOGO**  
(vosoritide) for injection

# Children with achondroplasia on VOXZOGO grew faster than without treatment<sup>1,42,43</sup>

## CANOPY ACH-3 (Study 301)

Phase 3 pivotal trial in children aged 5 to 15 years (N=121)<sup>36,39</sup>

### Mean ( $\pm$ SD) AGV at week 52<sup>42</sup>



AGV difference from placebo at 1 year in the primary analysis:

**+1.57 cm/yr<sup>1\*†‡</sup>**

*P*<0.0001 (95% CI: 1.22, 1.93)



AGV increase maintained over baseline at

**4 years**

in VOXZOGO-treated children from the open-label extension study (N=105)<sup>43</sup>

Mean ( $\pm$ SD) AGV at 4 years:  
VOXZOGO (n=49): 4.77 (2.17)

Placebo to VOXZOGO (n=56): 4.43 (2.35)

## Study design

- **CANOPY ACH-3 (Study 301):** Children aged 5 to 15 years were treated with VOXZOGO or placebo for 52 weeks.<sup>1,39</sup>
- **CANOPY ACH-EXT (Study 302):** Long-term efficacy and safety profile of VOXZOGO evaluated in children who continued treatment for up to 4 years in the open-label extension study.<sup>39,43</sup>

## Primary endpoint

- **Study 301:** Change from baseline in AGV at week 52 vs placebo. AGV is a measure of linear growth expressed as change in height or length over 1 year.<sup>1</sup>

**AGV**, annualized growth velocity; **CI**, confidence interval; **LS**, least squares; **SD**, standard deviation.

\*Improvement in AGV was consistent across all predefined subgroups, including sex, age, and Tanner stage.<sup>1</sup>

†All randomized subjects. Two patients in the VOXZOGO group discontinued from the study before Week 52; the values for these 2 patients were imputed assuming baseline growth rate for the period with missing data.<sup>1</sup>

‡Difference in LS mean change from baseline (VOXZOGO minus placebo; 2-sided *P*-value). LS means were estimated from the ANCOVA (analysis of covariance) model, which included treatment, stratum defined by sex and Tanner stage, baseline age, baseline AGV, and baseline height Z-score.<sup>1</sup>

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



**Corinna**

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

**97% of children on VOXZOGO (58/60) in the phase 3 pivotal trial remained on treatment during the 1-year study<sup>1</sup>**

**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.

**VOXZOGO**  
(vosoritide) for injection

10+ years of clinical trial  
experience in achondroplasia<sup>2</sup>

Evelyn

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





## Additional preliminary data continue to be collected

VOXZOGO 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).<sup>1</sup>

The following information is being provided to inform healthcare providers on the ongoing assessment and experience of patients in the clinical trial program.

**These data are not included in the US Prescribing Information and do not establish a clinical benefit or conclusions on efficacy. The analyses are preliminary and exploratory and should be interpreted cautiously.**

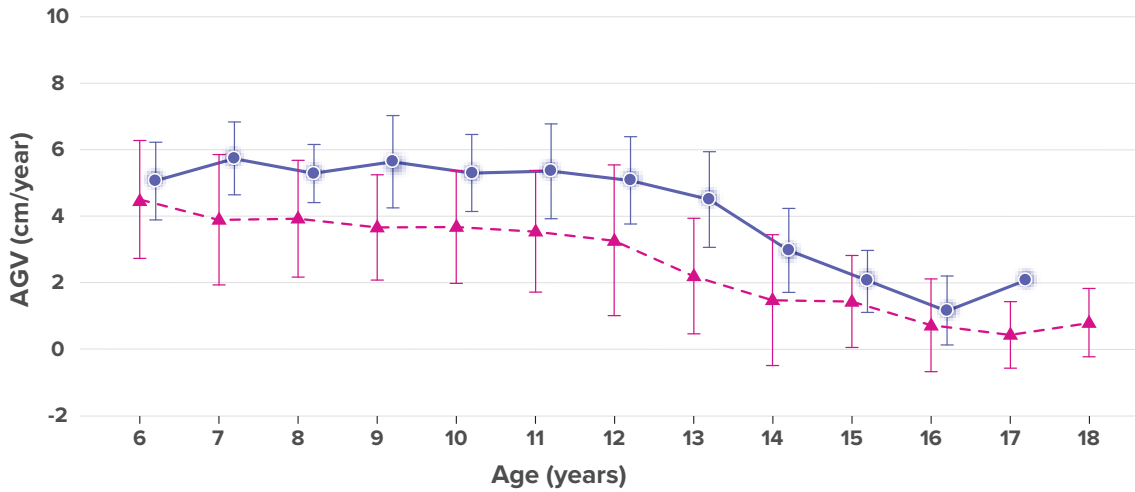
**Impact on final adult height has not been established  
and continues to be evaluated as studies are ongoing**

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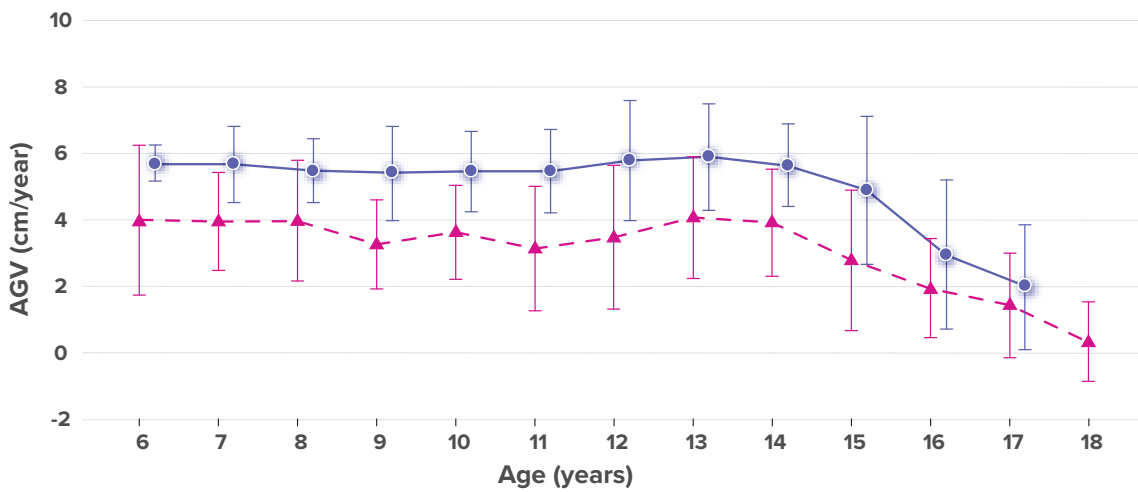
# AGV by age in VOXZOGO-treated children was compared to a separate untreated control group<sup>44</sup>

Mean ( $\pm$ SD) female AGV by age<sup>44\*</sup>



—▲— ACH Untreated	102	82	77	72	61	48	37	39	33	26	17	13	6
—●— ACH Treated	10	18	23	26	22	20	26	23	16	6	2	1	0

Mean ( $\pm$ SD) male AGV by age<sup>44\*</sup>



—▲— ACH Untreated	117	88	79	62	62	52	45	35	24	20	21	19	8
—●— ACH Treated	9	21	26	28	28	27	22	22	15	11	7	4	0

ACH, achondroplasia; AGV, annualized growth velocity; EXT, extension; SD, standard deviation.

\*Adapted from Savarirayan R, et al. *Med.* 2025;6(5):100566.<sup>44</sup>

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



Cal

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

## Data Limitations

- VOXZOGO-treated and untreated groups are from different studies; compare with caution as study design and methods may differ.<sup>44</sup>
- Effect of VOXZOGO on final adult height not yet established (continually assessing).<sup>44</sup>
- Preliminary results not in the US label, reflect a cross-sectional mix of treatment durations, are descriptive, may result from chance.<sup>1,44</sup>

## Study Design

- **CANOPY ACH-EXT (Study 302)** primary endpoints include AGV by age and sex in VOXZOGO-treated children.<sup>39,44</sup>
- Cohort: Full analysis set; age 5 to <18 years (N=119).<sup>44</sup>
- Mean (SD) treatment duration: 4.0 (0.8) years (min: 1.7 years, max: 6.2 years).<sup>44</sup>
- AGV derived for height assessments 12 ± 3 months apart and linked to a specific age integer considering the age at the midpoint of the 12-month interval.<sup>44</sup>
- **External ACH natural history study (CLARITY):** AGV of untreated children with ACH from CLARITY (age- and sex-matched to VOXZOGO-treated children) are plotted.<sup>44</sup>

## IMPORTANT SAFETY INFORMATION

### Pediatric Patients 0 to <5 Years:

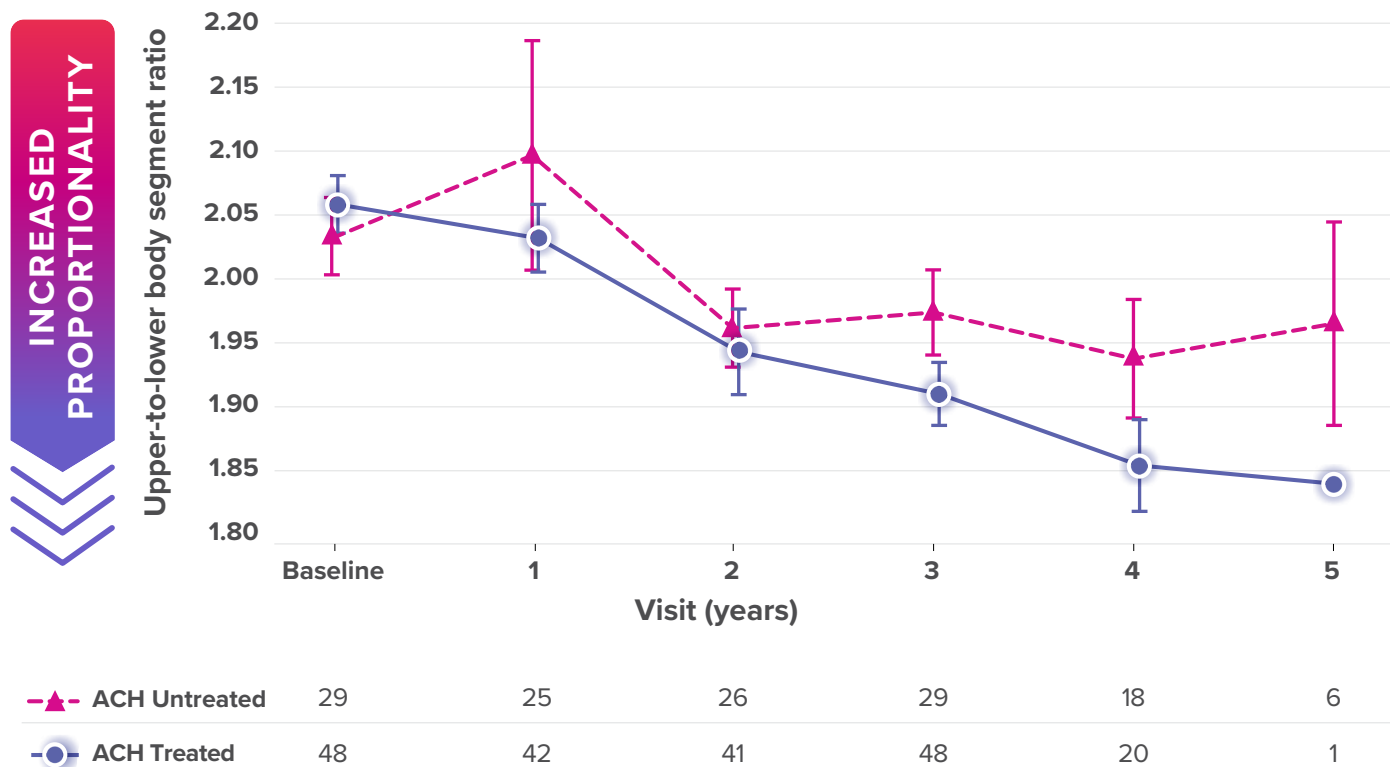
The safety of VOXZOGO in pediatric patients 0 to <5 years with achondroplasia was evaluated in a 52-week randomized, double-blind, placebo-controlled study (Study 2). In this study, 64 patients from birth to <5 years of age were randomized to receive either a daily vosoritide dose with similar exposure 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.

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# Body proportionality was measured over 5 years in a subset of children treated with VOXZOGO<sup>44</sup>

**CANOPY ACH-3 (Study 301):** After 1 year in the pivotal trial, the secondary endpoint of LS mean change from baseline in upper-to-lower body segment ratio was -0.02 in the placebo group (n=61) and -0.03 in the VOXZOGO group (n=58). The difference in LS mean change from baseline was -0.01 (95% CI: -0.05, 0.02; P=0.5).<sup>1,39,45</sup>

Mean ( $\pm$ SE) upper-to-lower body segment ratio<sup>44\*</sup>



ACH, achondroplasia; CI, confidence interval; LS, least squares; SE, standard error.

\*Adapted from Savarirayan R, et al. *Med.* 2025;6(5):100566.<sup>44</sup>

†In average-stature children, average upper-to-lower body segment ratio is 1.7 at birth and decreases to 1.0 at 10 years old. In ACH, the ratio never reaches 1.0 but still declines from birth up to ~age 10 years in girls and 11 years in boys.<sup>44</sup>

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



## Vihaan

7 YEARS OLD

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

### 5-Year Data Limitations

- VOXZOGO-treated and untreated groups are from different trials; compare with caution as study design and methods may differ.<sup>44</sup>
- Effect of VOXZOGO on final adult height and proportionality not yet established (continually assessing).<sup>44</sup>
- Preliminary results not in the US label, are descriptive, reflect small samples, may result from chance.<sup>1,44</sup>

### Study Design

- **CANOPY ACH-EXT (Study 302)** exploratory endpoints include body proportionality measured by upper-to-lower body segment ratio in VOXZOGO-treated children.<sup>39,44,46</sup>
- Cohort: Children with assessments at age <11 years (girls) or <12 years (boys); all children were ≥5 years old at VOXZOGO initiation. Assessments beyond these ages are excluded from analysis.<sup>44,46†</sup>
- **CANOPY ACH-OS (Study 901)** untreated arm and **CANOPY ACH-3 (Study 301)** placebo arm: Upper-to-lower body segment ratio of untreated children with ACH (age-matched to VOXZOGO-treated children) are plotted.<sup>39,44,46</sup>

### IMPORTANT SAFETY INFORMATION

#### 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.

**VOXZOGO**<sup>®</sup>  
(vosoritide) for injection

# The VOXZOGO clinical trial program continues to evaluate multiple endpoints<sup>1,2,40,45,47,48</sup>

VOXZOGO 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).<sup>1</sup>

As clinical trials are ongoing, the impact on final adult height has not been established and continues to be evaluated along with other pre-specified outcome measures.<sup>2,40,45,47,48</sup>

CANOPY ACH-3 (Study 301)

CANOPY ACH-EXT (Study 302)

CANOPY ACH-2I (Study 206)

CANOPY ACH-EXT (Study 208)

Secondary outcome measures include<sup>39,40,45\*</sup>:

- Functional measures (change from baseline)
- Bone morphology and quality
- Final adult height

Secondary outcome measures include<sup>39,47,48\*</sup>:

- Functional measures (change from baseline)
- Tibial bowing
- Spine morphology (eg, spinal canal width)
- Spinal alignment (eg, angles)
- Skull morphology (eg, foramen magnum area)

\*Visit [clinicaltrials.gov](https://clinicaltrials.gov) for the complete list of the trials' secondary outcome measures (NCT03197766, NCT03424018, NCT03583697, NCT03989947).<sup>40,45,47,48</sup>



Vivan

18 YEARS OLD

started VOXZOGO at 8 years old until growth plates closed as part of a clinical trial

## IMPORTANT SAFETY INFORMATION

### 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.

# Get to know the families who chose VOXZOGO

**Annika**

**14 YEARS OLD**

**Started VOXZOGO:** 9 years old as part of a clinical trial



Annika, 6 years old



Annika, 14 years old

For illustrative purposes only, image scale is not matched.



**Hear more stories from families who started and stayed on treatment**

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

**VOXZOGO**<sup>®</sup>  
(vosoritide) for injection

# The safety profile of VOXZOGO has been carefully evaluated<sup>1,44</sup>

## Children 5 to 15 years old

Over 1 year in the phase 3 pivotal trial (Study 301), adverse reactions that occurred in  $\geq 5\%$  of patients aged 5 to 15 years treated with VOXZOGO and at a percentage greater than placebo<sup>1</sup>:

ADVERSE REACTIONS <sup>1*</sup>	PLACEBO (n=61)	VOXZOGO (n=60)
Injection site erythema <sup>†</sup>	42 (69%)	45 (75%)
Injection site swelling <sup>†</sup>	22 (36%)	37 (62%)
Vomiting	12 (20%)	16 (27%)
Injection site urticaria <sup>†</sup>	6 (10%)	15 (25%)
Arthralgia	4 (7%)	9 (15%)
Decreased blood pressure	3 (5%)	8 (13%)
Gastroenteritis <sup>‡</sup>	5 (8%)	8 (13%)
Diarrhea	2 (3%)	6 (10%)
Dizziness <sup>§</sup>	2 (3%)	6 (10%)
Ear pain	3 (5%)	6 (10%)
Influenza	3 (5%)	6 (10%)
Fatigue <sup>  </sup>	2 (3%)	5 (8%)
Seasonal allergy	1 (2%)	4 (7%)
Dry skin	0 (0%)	3 (5%)

- In the long-term open-label extension study (Study 302), adverse reactions reported for up to 6 years of treatment are consistent with those seen in the 1-year pivotal trial.<sup>44</sup>

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 be well fed and hydrated in the hour before receiving VOXZOGO.<sup>1</sup>

## Children under 5 years old

The overall safety profile of VOXZOGO in pediatric patients <5 years was similar to that seen in older pediatric patients.<sup>1</sup>

- The most common adverse reactions (>10%) reported in pediatric patients <5 years were injection site reactions (86%) and rash (28%)<sup>1</sup>

\*Includes adverse reactions occurring more frequently in the VOXZOGO arm and with a risk difference of  $\geq 5\%$  (ie, difference of >2 subjects) between treatment arms.<sup>1</sup>

<sup>†</sup>Injection site reactions occurring more frequently in VOXZOGO-treated patients than placebo.<sup>1</sup>

<sup>‡</sup>Includes the preferred terms: gastroenteritis and gastroenteritis, viral.<sup>1</sup>

<sup>§</sup>Includes the preferred terms: dizziness, presyncope, procedural dizziness, and vertigo.<sup>1</sup>

<sup>||</sup>Includes the preferred terms: fatigue, lethargy, and malaise.<sup>1</sup>



Eve

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

## Administering VOXZOGO

VOXZOGO is a once-daily subcutaneous injection, administered at home by a trained caregiver.<sup>1</sup>

### Make sure to:



Monitor and assess patient body weight, growth, and physical development every 3-6 months and adjust the dosage according to the patient's actual body weight<sup>1</sup>



Discontinue VOXZOGO upon confirmation of no further growth potential, indicated by closure of growth plates<sup>1</sup>

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

**VOXZOGO**<sup>®</sup>  
(vosoritide) for injection

# Helping your patients start and stay on VOXZOGO<sup>1,35</sup>

VOXZOGO is a once-daily subcutaneous injection that is administered at home by trained caregivers.<sup>1</sup>

**You play an important role in helping patients and their families throughout the duration of treatment<sup>1,35</sup>**



## Identify goals

Talk about what they hope to get out of treatment; this may include growth as well as other personal goals.<sup>35</sup>



## Set appropriate expectations

**Response to VOXZOGO can vary and is often measurable after 1-2 years of starting treatment.** Ensure patients and their families understand potential treatment outcomes and effects to create realistic expectations.<sup>1,35\*</sup>



## Revisit their reason

Help remind them why they began treatment and reflect on their goals as they move forward through their journey.<sup>35</sup>

\*This is a select recommendation from the *International consensus guidelines on the implementation and monitoring of vosoritide therapy in individuals with achondroplasia*. The guidelines publication should be reviewed in its entirety along with the VOXZOGO Prescribing Information.<sup>1,35</sup>

## IMPORTANT SAFETY INFORMATION

### **Special Populations: (cont'd)**

- 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 m<sup>2</sup>. VOXZOGO is not recommended for patients with eGFR  $< 60$  mL/min/1.73 m<sup>2</sup>.

# Clinical Coordinators are here to help



## One-to-one support for:

- Providing product education and support around the injection process
- Helping to find an injection routine that works for your patients and their family throughout their 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

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

**VOXZOGO**  
(vosoritide) for injection

5,000+ infants and children with achondroplasia  
around the world are being prescribed VOXZOGO<sup>3</sup>



**Vihaan**

7 YEARS OLD

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



**Connect your patients with an experienced caregiver**

The VOXZOGO Mentor Program pairs your patients with a caregiver raising a child on VOXZOGO.

# Personalized assistance for families

## Widespread coverage to help you get started



OVER  
**96%**

of insured patients have secured  
coverage for **VOXZOGO**<sup>49\*</sup>



OVER  
**96%**

of eligible families with commercial insurance  
paid **\$0 out-of-pocket** for **VOXZOGO**<sup>50\*†</sup>



### 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 for eligible commercially insured patients<sup>†</sup>
- Offering guidance on insurance changes
- Ensuring the specialty pharmacy receives the prescription
- Educating your patient's care team on coverage requirements for continued access to therapy

\*BioMarin RareConnections™ Data on file. Patients included are eligible VOXZOGO patients who have enrolled in BioMarin RareConnections and are on commercial therapy.<sup>49,50</sup>

<sup>†</sup>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://www.biopharm.com/biomarin-copay-terms) for full Terms and Conditions.

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

**VOXZOGO**<sup>®</sup>  
(vosoritide) for injection

# 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.

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.

## **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.

**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.

## **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  $\geq 5$  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 m<sup>2</sup>. VOXZOGO is not recommended for patients with  $eGFR < 60$  mL/min/1.73 m<sup>2</sup>.

You may report side effects to the FDA at **1-800-FDA-1088** or [www.fda.gov/medwatch](http://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](#).



**Annika**

**14 YEARS OLD**

**on VOXZOGO since  
9 years old as part  
of a clinical trial**

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## Questions about VOXZOGO?

Contact your BioMarin representative  
to get the conversation started.

**VOXZOGO**<sup>®</sup>  
(vosoritide) for injection

# VOXZOGO is the only targeted therapy FDA approved from birth for children with achondroplasia<sup>1</sup>

Indicated to increase linear growth in children with achondroplasia until growth plates close.<sup>1</sup>



Target the underlying cause of inhibited endochondral bone growth.<sup>1,4,5</sup>



Promote a statistically significant increase in growth.<sup>1\*</sup>  
Growth increase was maintained over baseline at 4 years.<sup>1,4,3</sup>

\*Increase in annualized growth velocity vs placebo.<sup>1</sup>



Maximize time on treatment by starting early and continuing until growth plates close.<sup>1,6,35</sup>

BioMarin has been committed to skeletal conditions for 25 years, with over 10 years of VOXZOGO clinical trial experience in achondroplasia<sup>2,51</sup>

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(vosoritide) for injection

Scan the QR code or go to [VOXZOGO.com/HCP](https://VOXZOGO.com/HCP) to enroll your patients



## IMPORTANT SAFETY INFORMATION

### Warnings and Precautions for Risk of Low Blood Pressure

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