For HCPs treating with VOXZOGO[1]

Approved from birth. Backed by experience.

5,000+ infants and children with achondroplasia have been prescribed VOXZOGO worldwide[1-3]

VOXZOGO®
(vosoritide) for injection

The first and only treatment FDA approved from birth until growth plates close to increase linear growth for 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.

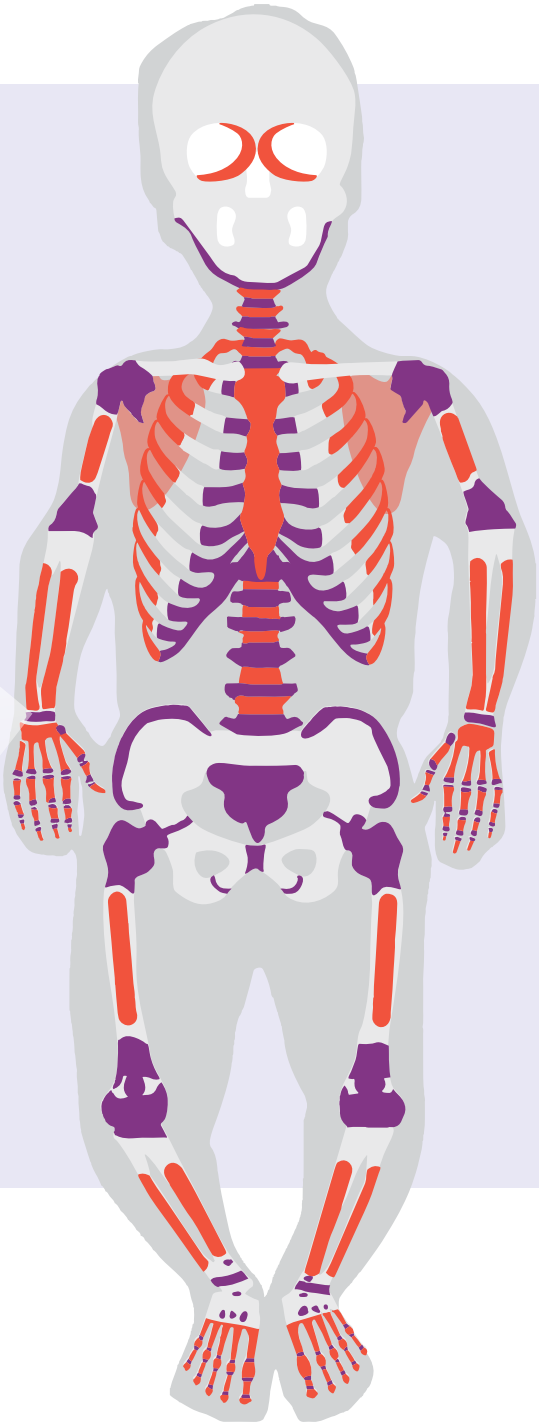
Endochondral bone growth is inhibited in achondroplasia[4,5]

Endochondral bone growth is the predominant process of bone development in which cartilage is replaced by bone at open growth plates.[6,7]

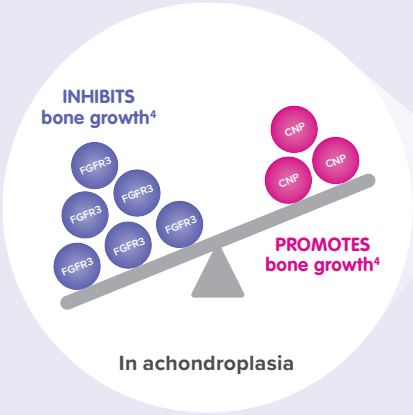
Endochondral bones make up

>90% of the bones in the body[7-16]

Endochondral bones Growth plates[6,9,17-28]



Overactive FGFR3 signaling relative to CNP signaling in growth plate cells is the underlying cause of inhibited bone growth in achondroplasia. Endogenous CNP levels cannot adequately regulate overactive FGFR3 signaling.[4]



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 in the full Prescribing Information.

Achondroplasia is associated with potential multisystemic skeletal complications[29]


Narrow nasopharyngeal airways


Foramen magnum stenosis


Spinal alignment


Spinal stenosis


Limited elbow extension and arm span


Disproportionate growth


Leg bowing

Many complications are **more common in the first 2 years of life**[5,29-31]

VOXZOGO has been approved to increase linear growth in pediatric patients with achondroplasia and open growth plates.[1] **Proactive and multidisciplinary care can help inform management and treatment approaches, which are important for multisystemic complications.**[5,32]

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.

Inhibited bone growth throughout the body can lead to reduced and disproportionate growth[4-7,30]

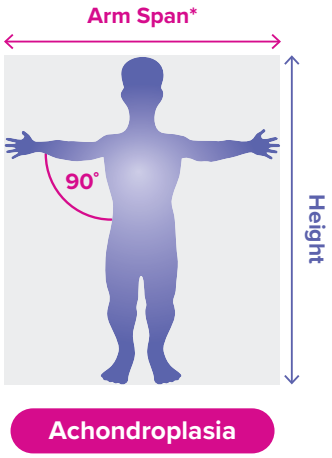
Disproportionate growth (differences in body proportions) is a characteristic feature of achondroplasia, which is associated with reduced mobility, pain, and dependence on devices and caregiver assistance.[30,33]

Achondroplasia affects how body proportions change over time, which is commonly measured by arm span, arm span-to-height ratio, and upper-to-lower body segment ratio.[33-36]

Arm span in adults with achondroplasia is ~35% shorter than average stature[33]

Arm span-to-height ratio: Arm span divided by standing height.[36]

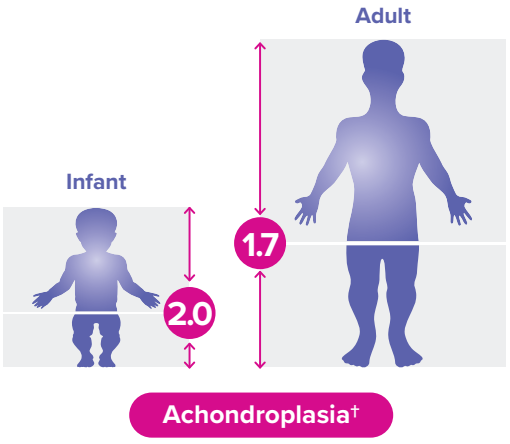
- In average-stature individuals, arm span is shorter than height before puberty and greater than height after mid-puberty[36]
- In achondroplasia, arm span is shorter than height before and after puberty (deficit increasing with age)[33,36]



Upper body remains longer than the lower body in adults with achondroplasia[34,35]

Upper-to-lower body segment ratio: Length of the upper body divided by the length of the lower body.[36]

- In average-stature individuals, upper body length at birth is greater than lower body length (ratio >1) but becomes proportional at 10 years old (ratio=1)[34,35]
- In achondroplasia, upper body length at birth is greater than lower body length (ratio >1) but remains disproportionate into adulthood (ratio >1)[34,35]



*Length between tips of the left and right middle fingers when standing against a flat wall with arms outstretched, creating a 90-degree angle with the torso.[36]

†Ratios represent the 50th percentile of children with achondroplasia.[34,35]

Please see additional Important Safety Information presented throughout, and in the full Prescribing Information.

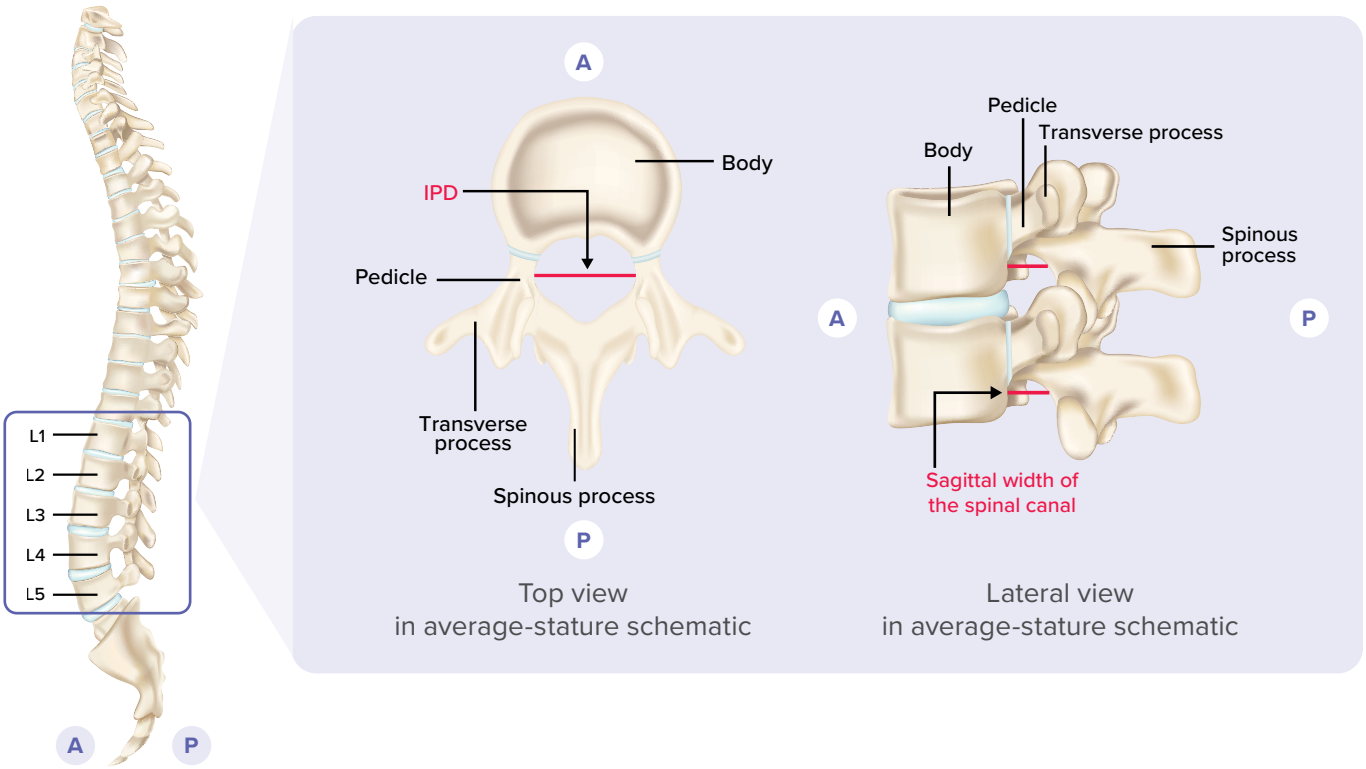
Achondroplasia is associated with the development of spinal stenosis[29]

Spinal stenosis (narrowing of the spinal canal that compresses the spinal cord/nerve roots) in the lumbar spine is often symptomatic in adulthood, associated with chronic pain, walking intolerance, bowel/bladder dysfunction, and paraplegia.[29,37,38]

However, its development can begin much earlier in children with achondroplasia, given that their spinal canal reaches ~95% of adult size before 5 years old.[37,38]

Common spinal canal measures related to spinal stenosis[37]:

- Narrowing of **interpedicular distance (IPD)** on A/P X-ray
- Reduced **sagittal width of the spinal canal** on lateral X-ray



A, anterior (front); **L1-5,** lumbar vertebra 1-5; **P,** posterior (back).

IMPORTANT SAFETY INFORMATION

Adverse Reactions:

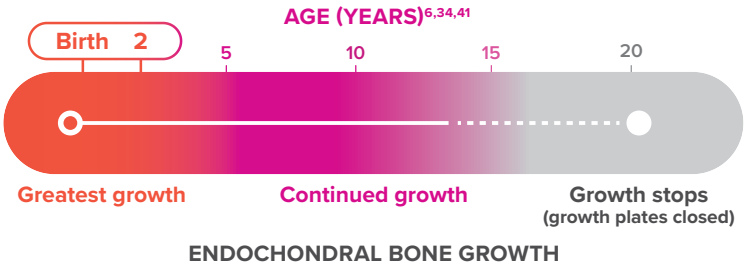
Adverse reactions that occurred in ≥5% of patients treated with VOXZOGO and at a rate greater than that of placebo in the phase 3 study are injection site reactions (including erythema, swelling, urticaria, pain, bruising, pruritus, hemorrhage, discoloration, and induration), vomiting, arthralgia, decrease 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.

Only VOXZOGO is FDA approved to promote bone growth as early as birth[1]

VOXZOGO improves the balance of cell signaling by mimicking the body's natural CNP to increase linear growth in children with achondroplasia—only while growth plates remain open.[1]

The most rapid growth occurs in the first 2 years of life[34,39]

By 2 years old, children with achondroplasia reach more than half their adult height[40]*



ENDOCHONDRAL BONE GROWTH

International consensus guidelines support early initiation of VOXZOGO[42]

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 the 2025 publication for additional detail.[42]

SCAN THE QR CODE TO LEARN MORE

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

*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.[40]

Please see additional Important Safety Information presented throughout, and in the full Prescribing Information.

VOXZOGO® (vosoritide) for injection

VOXZOGO has been studied in children aged 4 months to <18 years[1,43-45]

CANOPY ACH-OS (Study 901)
Ongoing Observational Study[45,46]

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

CANOPY ACH-2I (Study 206)†
Phase 2 Infant and Toddler Study[43,46]

- Age: 4 months to <5 years
- 52-week, double-blind, randomized, placebo-controlled

CANOPY ACH-EXT (Study 208)
Ongoing Open-label Extension Study[46,47]

- Duration: Near final adult height

CANOPY ACH-3 (Study 301)
Phase 3 Pivotal Study[44,46]

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

CANOPY ACH-EXT (Study 302)
Ongoing Open-label Extension Study[44,46,48]

- Duration: Final adult height

Data continue to be collected in ongoing clinical and real-world studies[2]

ACH, achondroplasia; **EXT,** extension; **OS,** observational study.

*Duration of ≥6 months if aged ≥3 months at study entry; duration of ≥3 months if aged <3 months at study entry.[45]

†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.[43,46]

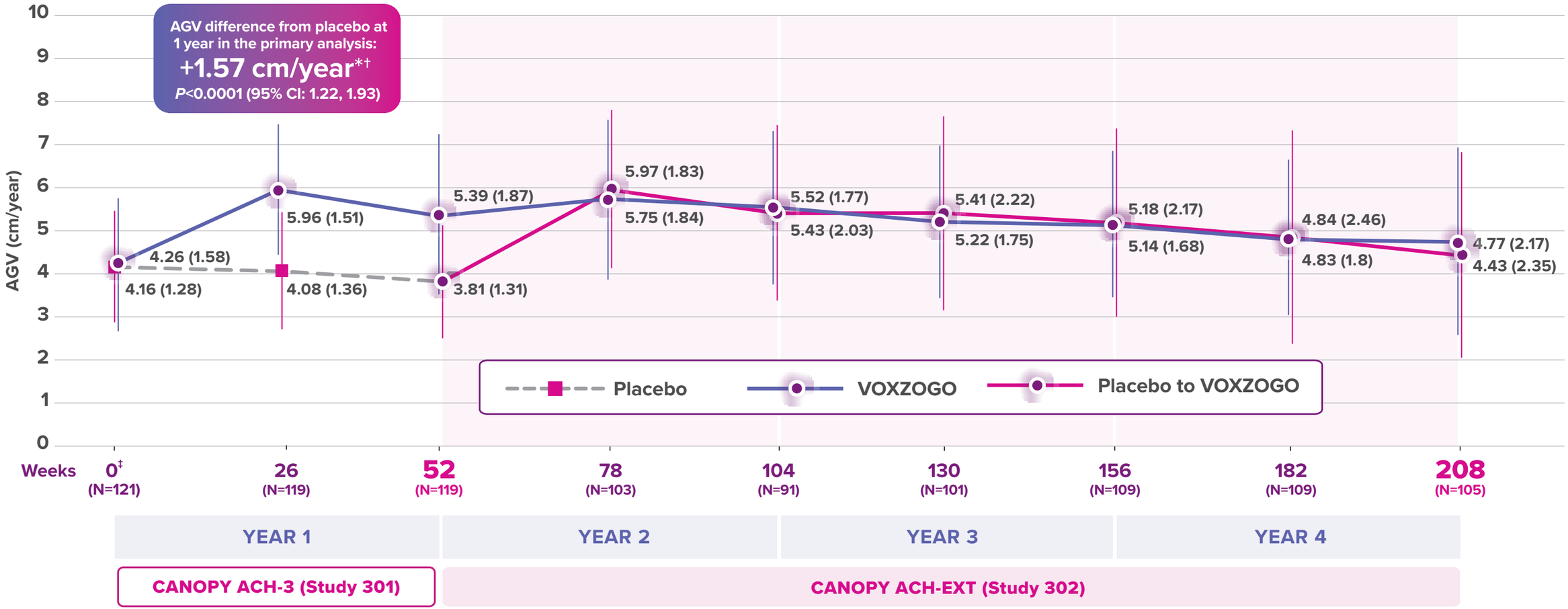
IMPORTANT SAFETY INFORMATION

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.

Children with achondroplasia on VOXZOGO grew faster than without treatment[1,49]

Mean (±SD) AGV over time in children aged 5 to 15 years[1,46,49]



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.

Please see additional Important Safety Information presented throughout, and in the full Prescribing Information.

VOXZOGO® (vosoritide) for injection

97% of children on VOXZOGO (58/60) remained on treatment during the 1-year pivotal trial[1]

Study Design

- **CANOPY ACH-3 (Study 301):** Children aged 5 to 15 years were treated with VOXZOGO or placebo for 52 weeks[1,46]
- **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[46,49]

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[1]

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

*All randomized subjects. 2 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.[1]

†Difference in LS mean change from baseline. LS means estimated from the ANCOVA (analysis of covariance) model, which included treatment, stratum by sex and Tanner stage, baseline age, baseline AGV, and baseline height Z-score.[1]

‡Baseline AGV was established in the run-in observational Study 901, based on standing height measurements at least 6 months prior to enrollment in Study 301.[45]

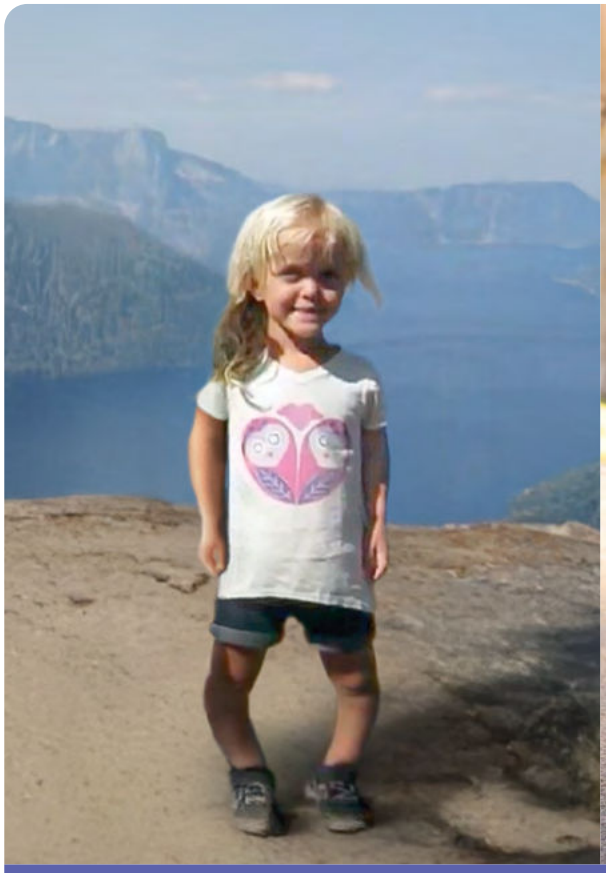
Please see additional Important Safety Information presented throughout, and in the full Prescribing Information.

An established history in achondroplasia[1-3]

Annika

14 YEARS OLD

Started VOXZOGO: 9 years old



Annika, 6 years old

Annika, 14 years old

For illustrative purposes only, image scale is not matched.

10+ years of clinical trial experience in achondroplasia and 4+ years of real-world use[1,2]

Please see additional Important Safety Information presented throughout, and in the full Prescribing Information.

VOXZOGO® (vosoritide) for injection

Additional preliminary data continue to be collected[1,2,47,50-52]

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).[1]

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.

- **Additional Growth Measures**
- **Body Proportionality**
- **Orthopedic Measures**
- **Ongoing Studies**

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

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 was studied in children aged 4 months to <5 years[43]

CANOPY ACH-OS (Study 901)

Ongoing Observational Study[45,46]

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

CANOPY ACH-2I (Study 206)†

Phase 2 Infant and Toddler Study[43,46]

- Age: 4 months to <5 years
- 52-week, double-blind, randomized, placebo-controlled

CANOPY ACH-EXT (Study 208)

Ongoing Open-label Extension Study[46,47]

- All participants on VOXZOGO
- Duration: Near final adult height

Study Design (Study 206)

- Children with ACH aged 4.4 to 59.8 months old at treatment initiation with VOXZOGO (n=32 randomized; n=11 sentinels) or placebo (n=32 randomized)[43]
  - Observational run-in study (Study 901 or Study 206) established baseline AGV[43]*
- No formal sample-size calculations were made to power the study; predefined statistical analyses are descriptive[43]

Co-primary Endpoints (Study 206)

- **Safety and tolerability of VOXZOGO**[43,52]
- **Change from baseline in height Z-score at 52 weeks:** After 1 year, difference in LSM height Z-score change from baseline with VOXZOGO (n=32) relative to placebo (n=32) was 0.25 (95% CI: -0.02 to 0.53)[43,52†‡]

ACH, achondroplasia; **AGV,** annualized growth velocity; **CDC,** Centers for Disease Control and Prevention; **CI,** confidence interval; **EXT,** extension; **LSM,** least squares mean; **OS,** observational study.

*Duration of ≥6 months if aged ≥3 months at study entry; duration of ≥3 months if aged <3 months at study entry.[45]

†Average-stature referenced height Z-scores converted from standing height using age- and sex-matched CDC reference data for average-stature children; if <24 months at baseline, body length took precedence over standing height.[43,53]

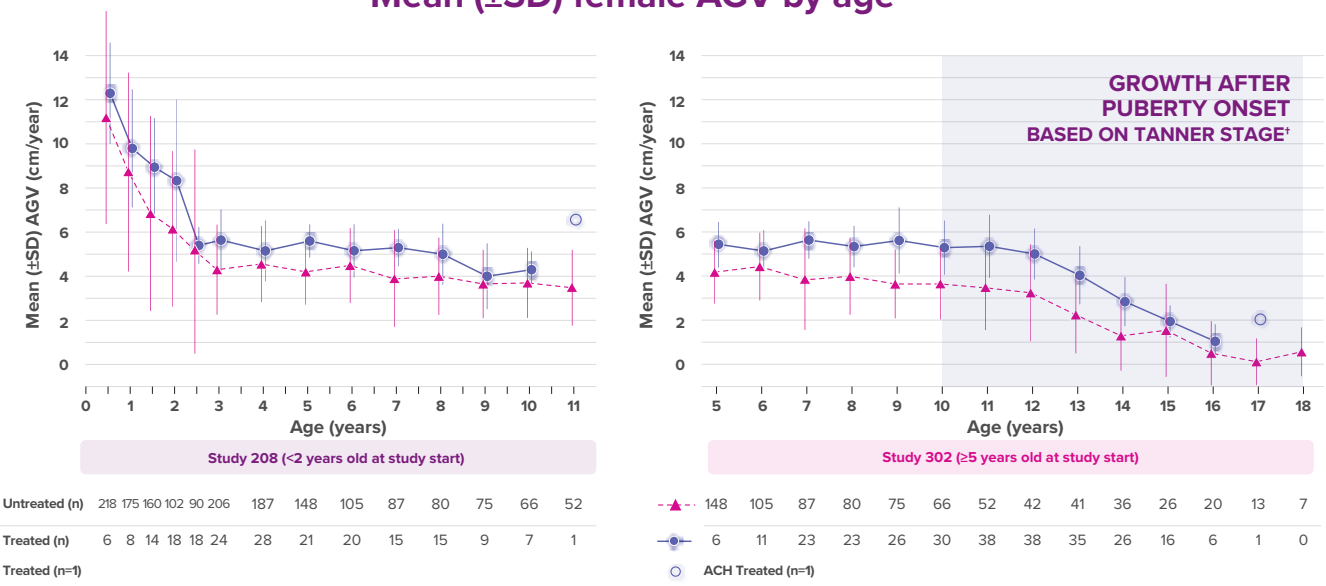
‡Difference in LSM change from baseline: VOXZOGO minus placebo. LSM (95% CI) and LSM differences estimated from ANCOVA (analysis of covariance) model, including treatment arm, sex, randomization age strata, and baseline age, height Z-score, and AGV.[43,53]

Please see additional Important Safety Information presented throughout, and in the full Prescribing Information.

VOXZOGO® (vosoritide) for injection

AGV by age in VOXZOGO-treated children was compared to separate untreated control groups[54]*

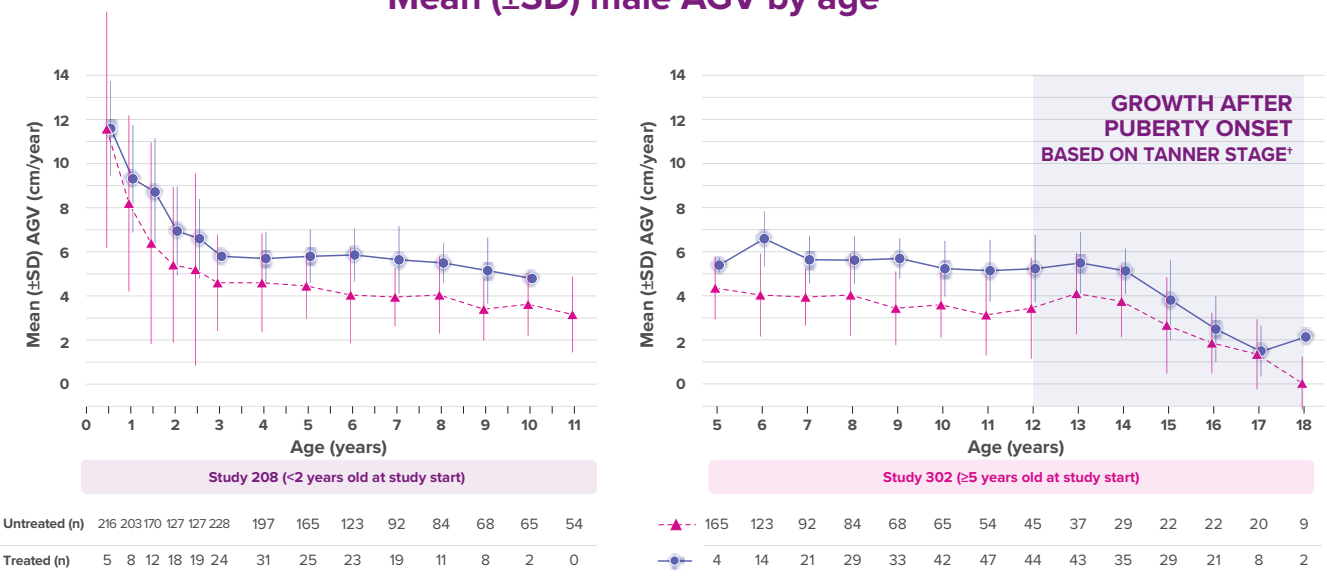
Mean (±SD) female AGV by age[46,55,56]



ACH Untreated (n)	218	175	160	102	90	206	187	148	105	87	80	75	66	52
ACH Treated (n)	6	8	14	18	18	24	28	21	20	15	15	9	7	1
ACH Treated (n=1)														

148	105	87	80	75	66	52	42	41	36	26	20	13	7
6	11	23	23	26	30	38	38	35	26	16	6	1	0
ACH Treated (n=1)													

Mean (±SD) male AGV by age[46,55,56]



ACH Untreated (n)	216	203	170	127	127	228	197	165	123	92	84	68	65	54
ACH Treated (n)	5	8	12	18	19	24	31	25	23	19	11	8	2	0

165	123	92	84	68	65	54	45	37	29	22	22	20	9
4	14	21	29	33	42	47	44	43	35	29	21	8	2

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

*AGV by age was assesed in Study 208 and Study 302, with an external ACH natural history study (CLARITY) used as separate untreated control groups.[54,58]

†In Study 302, the mean age of puberty onset was 10.7 years in females and 12.1 years in males; 26 females and 33 males were assessed for puberty onset as defined by the increase of any Tanner stage from 1 to 2 (ie, females: earliest increase in pubic hair or breast budding; males: earliest increase in pubic hair or testicular volume).[57]

‡For ages <3 years, AGV derived for height assessments 6 ± 1.5 months apart.[55]

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.

Data Limitations

- **Results are exploratory, are not in the US label, and should be interpreted with caution**[1,54]
- VOXZOGO-treated and untreated groups are from different studies; compare with caution as study design and methods may differ[54]
- Effect of VOXZOGO on final adult height not yet established (continually assessing); preliminary results reflect a cross-sectional mix of treatment durations, are descriptive, may result from chance[54]

Study Design

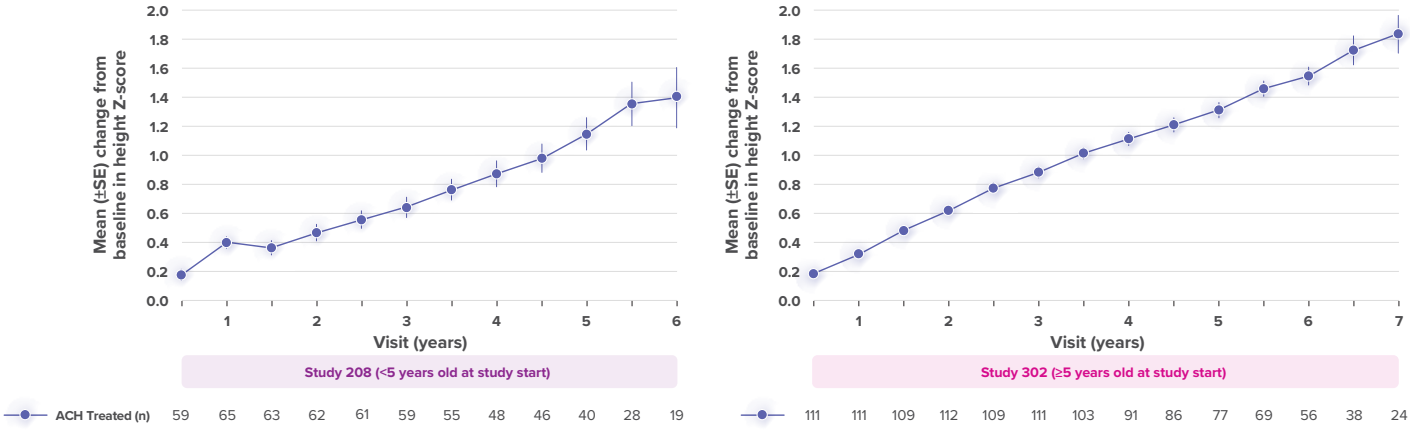
- **CANOPY ACH-EXT (Study 208 and Study 302)** secondary and primary endpoints include AGV by age and sex in VOXZOGO-treated children[46,54,58]
- Cohorts: Study 208, age <2 years (N=33), mean treatment duration (SD) for ages 0 to <0.5 years and 0.5 to <2 years: 4.5 (0.8) years and 4.8 (1.5) years; Study 302, age 5 to <18 years (N=119), mean treatment duration (SD): 4.0 (0.8) years[54,58]
- 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[55,56,58‡]
- **External ACH natural history study (CLARITY):** AGV of untreated children with ACH from CLARITY (age- and sex-matched to VOXZOGO-treated children) are plotted[54,58]

Please see additional Important Safety Information presented throughout, and in the full Prescribing Information.

VOXZOGO® (vosoritide) for injection

Height Z-score was measured over 7 years in VOXZOGO-treated children relative to untreated children[59]

Change from baseline in height Z-score (ACH referenced)[59]*



Height Z-scores increased from baseline with VOXZOGO[59]

ACH, achondroplasia; **EXT,** extension; **SD,** standard deviation; **SE,** standard error.

*Adapted from Savarirayan R, et al. PES. San Francisco, CA, USA. 2026. Poster 32.[59]

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 ≥60 mL/min/1.73 m^2. VOXZOGO is not recommended for patients with eGFR <60 mL/min/1.73 m^2.

Data Limitations

- **Results are exploratory, are not in the US label, and should be interpreted with caution**[1,59]
- VOXZOGO-treated and untreated children are from different studies; compare with caution as study design and methods may differ[59]
- Effect of VOXZOGO on final adult height not yet established (continually assessing); preliminary results are descriptive, may result from chance[59]

Study Design

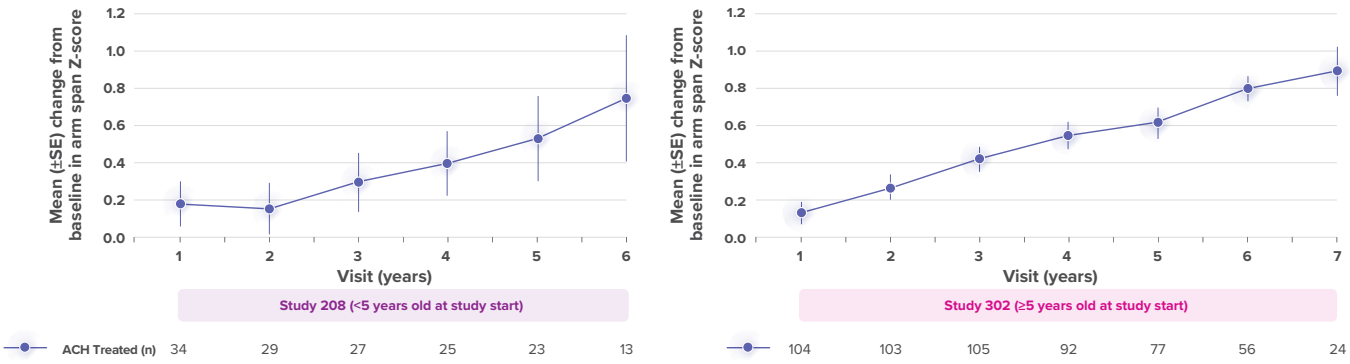
- **CANOPY ACH-EXT (Study 208 & Study 302)** primary endpoints include height Z-scores in VOXZOGO-treated children, assessed every 6 months[46,47,59,60]
- Cohorts: Study 208, age <5 years at VOXZOGO initiation (N=73), mean treatment duration (SD): 5.0 (1.5) years; Study 302, age 5 to <18 years at VOXZOGO initiation (N=119), mean treatment duration (SD): 5.2 (1.4) years[59]
- Untreated ACH referenced height Z-scores: Derived from height/body length using age- and sex-matched reference data of untreated children with ACH from an **external ACH natural history study (CLARITY)**[59,61]

Please see additional Important Safety Information presented throughout, and in the full Prescribing Information.

VOXZOGO® (vosoritide) for injection

Arm span Z-score was measured over 7 years in VOXZOGO-treated children relative to untreated children[59]

Change from baseline in arm span Z-score (ACH referenced)[59]*



Arm span Z-scores increased from baseline with VOXZOGO while maintaining arm span-to-height ratio across all years of treatment[59]

ACH, achondroplasia; **EXT,** extension; **SD,** standard deviation; **SE,** standard error.

*Adapted from Savarirayan R, et al. PES. San Francisco, CA, USA. 2026. Poster 32.[59]

IMPORTANT SAFETY INFORMATION

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.

Data Limitations

- **Results are exploratory, are not in the US label, and should be interpreted with caution**[1,59]
- VOXZOGO-treated and untreated children are from different studies; compare with caution as study design and methods may differ[59]
- Effect of VOXZOGO on final adult height not yet established (continually assessing); preliminary results are descriptive, may result from chance[59]

Study Design

- **CANOPY ACH-EXT (Study 208 & Study 302)** secondary endpoints include body proportionality measures (arm span, arm span-to-height ratio) in VOXZOGO-treated children, assessed every year[46,51,59,60]
- Cohorts: Study 208, age <5 years at VOXZOGO initiation (N=73), mean treatment duration (SD): 5.0 (1.5) years; Study 302, age 5 to <18 years at VOXZOGO initiation (N=119), mean treatment duration (SD): 5.2 (1.4) years[59]
- Untreated ACH referenced arm span Z-scores: Derived from age- and sex-matched reference data of untreated children with ACH from an **external ACH natural history study**[59,62]

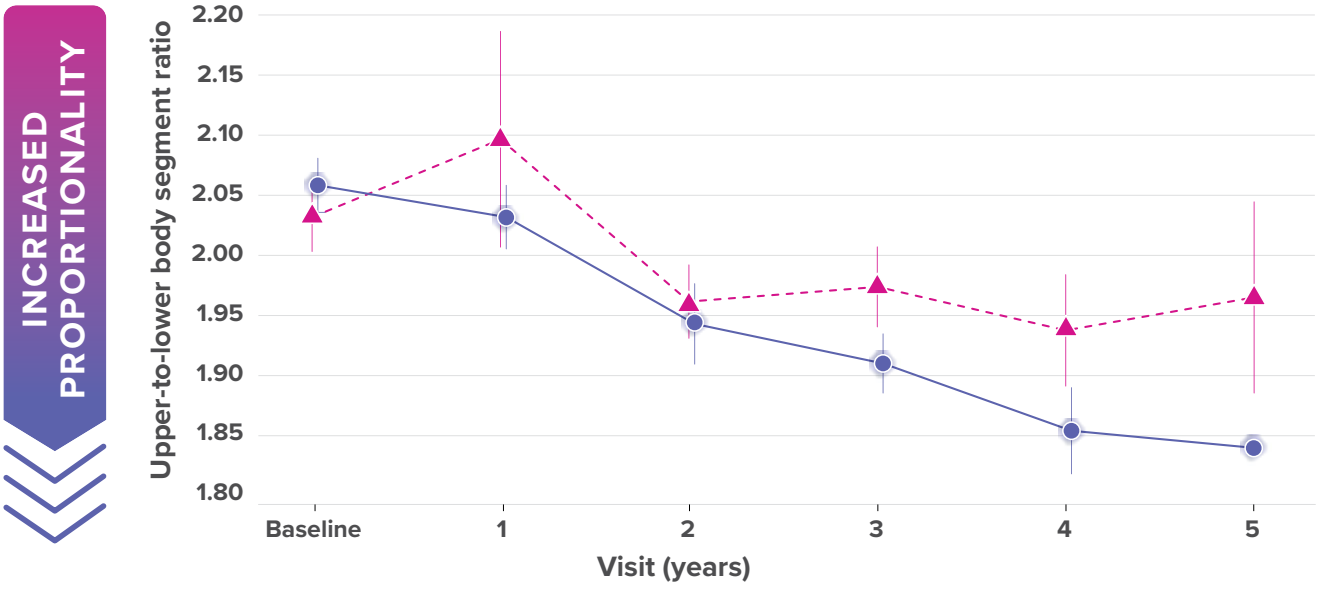
Please see additional Important Safety Information presented throughout, and in the full Prescribing Information.

VOXZOGO® (vosoritide) for injection

Upper-to-lower body segment ratio was measured over 5 years in a subset of VOXZOGO-treated children[58]

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).[1,46,50]

Mean (±SE) upper-to-lower body segment ratio[58]*



	Baseline	1	2	3	4	5
ACH Untreated (n)	29	25	26	29	18	6
ACH Treated (n)	48	42	41	48	20	1

ACH, achondroplasia; **CI,** confidence interval; **EXT,** extension; **LS,** least squares; **OS,** observational study; **SE,** standard error.

*Adapted from Savarirayan R, et al. *Med.* 2025;6(5):100566.[58]

†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.[58]

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.

5-Year Data Limitations

- **Results are exploratory, are not in the US label, and should be interpreted with caution**[1,58]
- VOXZOGO-treated and untreated groups are from different trials; compare with caution as study design and methods may differ[58]
- Effect of VOXZOGO on final adult height and proportionality not yet established (continually assessing); preliminary results are descriptive, reflect small samples, may result from chance[58]

Study Design

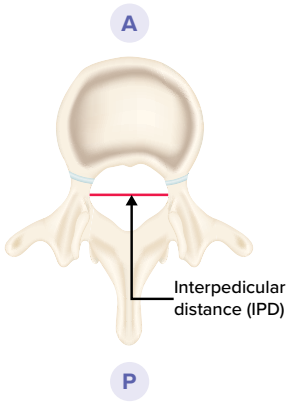
- **CANOPY ACH-EXT (Study 302)** exploratory endpoints include body proportionality measured by upper-to-lower body segment ratio in VOXZOGO-treated children[46,58,60]
- Cohort: Children with assessments at age <11 years (girls) or <12 years (boys); all children were ≥5 years old at VOXZOGO initiation. Assessments beyond these ages are excluded from analysis[58,60†]
- **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[46,58,60]

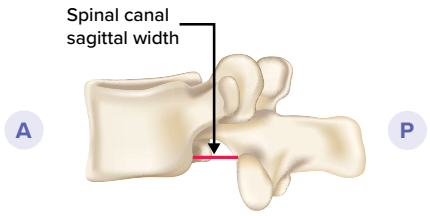
Please see additional Important Safety Information presented throughout, and in the full Prescribing Information.

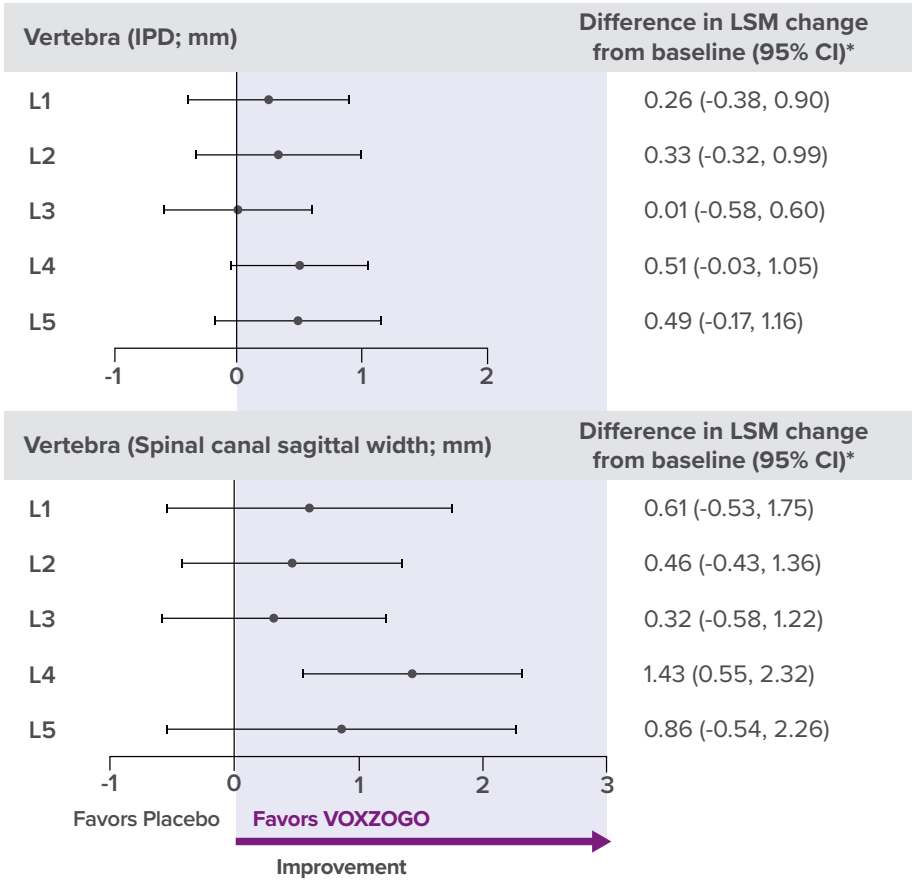
VOXZOGO® (vosoritide) for injection

At 1 year, change from baseline in spinal canal measures were evaluated in VOXZOGO-treated children <5 years old vs placebo[37]

Difference in change from baseline in interpedicular distance and spinal canal sagittal width at all lumbar vertebrae with VOXZOGO vs placebo[37]*







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.

Data Limitations

- **Results are exploratory, are not in the US label, and should be interpreted with caution**[1,37]
- Effect of VOXZOGO on final adult height not yet established (continually assessing); preliminary results are descriptive, reflect small samples, may result from chance[37]

Study Design

- **CANOPY ACH-2I (Study 206)** secondary endpoints include change from baseline in lumbar spine measures (IPD, spinal canal sagittal width) at 52 weeks in children treated with VOXZOGO vs placebo[37,46,52]
- Cohort: Individuals with ACH aged 4 months to <5 years at initiation of VOXZOGO (n=40) or placebo (n=27)[37]
- Spinal canal morphology: Assessed with A/P and lateral X-rays at baseline and 52 weeks with standardized image acquisition; IPD and spinal canal sagittal width for L1-5 were defined by independent radiologists[37]

A, anterior (front); **ACH,** achondroplasia; **AGV,** annualized growth velocity; **CI,** confidence interval; **IPD,** interpedicular distance; **LSM,** least squares mean; **L1-5,** lumbar vertebra 1-5; **P,** posterior (back).

*Difference in LSM change from baseline was calculated as VOXZOGO minus placebo. LSM differences (95% CI) between treatment groups estimated from ANCOVA (analysis of covariance) model, which included treatment arm, sex, randomization age strata, and baseline age, height Z-score, AGV, and morphology measures.[37]

Please see additional Important Safety Information presented throughout, and in the full Prescribing Information.

VOXZOGO® (vosoritide) for injection

The VOXZOGO clinical trial program continues to evaluate multiple endpoints[1,2,47,50-52]

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).[1]

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.[2,47,50-52]

CANOPY ACH-2I (Study 206) | **CANOPY ACH-EXT (Study 208)**

Secondary outcome measures include[46,47,52]*:

- **Functional & HRQOL measures (eg, ITQOL, WeeFIM-II)**
- **Tibial bowing**
- **Spine morphology**
- **Spinal alignment (eg, angles)**
- **Skull morphology (eg, foramen magnum area)**

CANOPY ACH-3 (Study 301) | **CANOPY ACH-EXT (Study 302)**

Secondary outcome measures include[46,50,51]*:

- **Functional & HRQOL measures (eg, QoLISSY, WeeFIM-II)**
- **Bone morphology and quality**
- **Final adult height**

ACH, achondroplasia; **EXT,** extension; **HRQOL,** health-related quality of life; **ITQOL,** Infant Toddler Quality of Life; **QoLISSY,** Quality of Life in Short Stature Youth; **WeeFIM-II,** Pediatric Functional Independence Measure, 2nd version.

*Visit clinicaltrials.gov for the complete list of the trials' secondary outcome measures (NCT03583697, NCT03989947, NCT03197766, NCT03424018).[47,50-52]

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.

5,000+ infants and children with achondroplasia have been prescribed VOXZOGO worldwide[1,3]

Hear more stories from caregivers and children who felt VOXZOGO was the right choice for their family[1]

Please see additional Important Safety Information presented throughout, and in the full Prescribing Information.

VOXZOGO® (vosoritide) for injection

The safety profile of VOXZOGO has been carefully evaluated from infancy through childhood[1,58,63]

Children 5 to 15 years old

Over 1 year in the phase 3 pivotal trial (Study 301), adverse reactions that occurred in ≥5% of patients aged 5 to 15 years treated with VOXZOGO and at a percentage greater than placebo[1]:

ADVERSE REACTIONS[1]*	PLACEBO (n=61)	VOXZOGO (n=60)
Injection site erythema†	42 (69%)	45 (75%)
Injection site swelling†	22 (36%)	37 (62%)
Vomiting	12 (20%)	16 (27%)
Injection site urticaria†	6 (10%)	15 (25%)
Arthralgia	4 (7%)	9 (15%)
Decreased blood pressure	3 (5%)	8 (13%)
Gastroenteritis‡	5 (8%)	8 (13%)
Diarrhea	2 (3%)	6 (10%)
Dizziness§	2 (3%)	6 (10%)
Ear pain	3 (5%)	6 (10%)
Influenza	3 (5%)	6 (10%)
Fatigue‖	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.[58]

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.[1]

*Includes adverse reactions occurring more frequently in the VOXZOGO arm and with a risk difference of ≥5% (ie, difference of >2 subjects) between treatment arms.[1]

†Injection site reactions occurring more frequently in VOXZOGO-treated patients than placebo.[1]

‡Includes the preferred terms: gastroenteritis and gastroenteritis, viral.[1]

§Includes the preferred terms: dizziness, presyncope, procedural dizziness, and vertigo.[1]

‖Includes the preferred terms: fatigue, lethargy, and malaise.[1]

Children under 5 years old

The overall safety profile of VOXZOGO in pediatric patients <5 years old was similar to that seen in older pediatric patients.[1]

- **Over 1 year in the phase 2 trial (Study 206),** the most common adverse reactions (>10%) reported in pediatric patients <5 years old were injection site reactions (86%) and rash (28%)[1]

Children under 2 years old

Adverse reactions reported in children 4 months to <2 years old were consistent with those seen in children aged 2 to <5 years old.[63]

In the long-term open-label extension study (Study 208), adverse reactions reported for up to 4 years of treatment are consistent with those seen in the 1-year trial.[63]

Please see additional Important Safety Information presented throughout, and in the full Prescribing Information.

VOXZOGO® (vosoritide) for injection

Administering VOXZOGO

VOXZOGO is a once-daily subcutaneous injection, administered at home by a trained caregiver.[1]

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.[1]

Discontinue VOXZOGO upon confirmation of no further growth potential, indicated by closure of growth plates.[1]

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.

Helping your patients start and stay on VOXZOGO[1,42]

You play an important role in helping patients and their families throughout the duration of treatment.[1,42]

Identify goals

Talk about what they hope to get out of treatment; this may include growth as well as other personal goals.[42]

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.[1,42]*

Revisit their reason

Help remind them why they began treatment and reflect on their goals as they move forward through their journey.[42]

*This is a select recommendation from the *International consensus guidelines on the implementation and monitoring of vosoritide therapy in individuals with achondroplasia*. The 2025 guidelines publication should be reviewed in its entirety along with the VOXZOGO Prescribing Information.[1,42]

VOXZOGO MENTOR PROGRAM

The VOXZOGO Mentor Program connects your patients' families with another caregiver raising a child with achondroplasia who understands their experience and can share personal insights or offer support.

Please see additional Important Safety Information presented throughout, and in the full Prescribing Information.

VOXZOGO® (vosoritide) for injection

Clinical Coordinators are here to help

BIOMARIN Clinical Coordinators

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 families 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

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

- Christina, BioMarin Clinical Coordinator

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.

Personalized assistance for families

Widespread coverage to help your patients get started

OVER 96% of insured patients have secured coverage for VOXZOGO[64*]

OVER 96% of eligible families with commercial insurance paid $0 out of pocket for VOXZOGO[65*†]

BioMarin Rare Connections™

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†
- Offering guidance on insurance changes
- Ensuring the specialty pharmacy receives the prescription
- Educating your patients' 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.[64,65]

†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 for full Terms and Conditions.

Please see additional Important Safety Information presented throughout, and in the full Prescribing Information.

VOXZOGO® (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 ≥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 ≥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 ≥60 mL/min/1.73 m^2. VOXZOGO is not recommended for patients with eGFR <60 mL/min/1.73 m^2.

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.

References: 1. VOXZOGO [package insert]. Novato, CA: BioMarin Pharmaceutical Inc; 2024. **2.** ClinicalTrials.gov. Search: Achondroplasia, BioMarin Pharmaceutical. Accessed May 14, 2026. https://clinicaltrials.gov/search?cond=Achondroplasia&term=BioMarin%20Pharmaceutical&viewType=Table **3.** Data on file [1]. BioMarin Pharmaceutical Inc; 2025. **4.** Horton WA, Hall JG, Hecht JT. Achondroplasia. *Lancet.* 2007;370(9582):162-172. **5.** Savarirayan 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. **6.** Mackie EJ, Tatarczuch L, Mirams M. The skeleton: a multi-functional complex organ: the growth plate chondrocyte and endochondral ossification. *J Endocrinol.* 2011;211(2):109-121. **7.** Clarke B. 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Questions about VOXZOGO?

Contact your BioMarin representative
to get the conversation started.

VOXZOGO®
(vosoritide) for injection

VOXZOGO is the first and only targeted therapy FDA approved from birth for children with achondroplasia[1]

Indicated to increase linear growth in children with achondroplasia until growth plates close.[1]

Promote a statistically significant increase in growth.[1]* Growth increase was maintained over baseline at 4 years.[1,49]

Prescribed to +5,000 infants and children with achondroplasia worldwide.[1,3]

Widespread coverage with >96% of eligible families securing access.[64†]

*Increase in annualized growth velocity vs placebo.[1]

†BioMarin RareConnections™ Data on file. Patients included are eligible VOXZOGO patients who have enrolled in BioMarin RareConnections and are on commercial therapy.[64]

VOXZOGO®
(vosoritide) for injection

Scan the QR code or go to VOXZOGO.com/HCP to enroll your patients

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.

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