



Managing achondroplasia with vosoritide (VOXZOGO[®]▼)

Information for caregivers

Learn about achondroplasia and treatment with vosoritide as part of a comprehensive management plan

This material is only for caregivers of patients who have been prescribed vosoritide. It is an additional information resource that should be read alongside the package leaflet. Always refer to the package leaflet before beginning treatment with vosoritide. If you have any questions, please discuss them with your child's doctor.

Reporting of side effects: If your child gets any side effects, talk to your child's doctor, or nurse. This includes any possible side effects not listed in the package leaflet. By reporting side effects you can help provide more information on the safety of this medicine.

▼ This medicinal product is subject to additional monitoring. This will allow quick identification of new safety information. You can help by reporting any side effects directly to BioMarin via drugsafety@bmrn.com; Fax: +1-415-532-3144 or Tel: +1-415-506-6179. Reporting of side effects is also possible via national reporting systems.

Suspected Adverse Drug Reactions (side effects) or medication errors may be reported using the Medicines Authority ADR reporting form, which is available online at <http://www.medicinesauthority.gov.mt/adrportal>, and sent by post or email to; P: Pharmacovigilance Section at Post-Licensing Directorate, Medicines Authority, Sir Temi Zammit Buildings, Malta Life Sciences Park, San Ġwann SĠN 3000 E: postlicensing.medicinesauthority@gov.mt

B:OMARIN[®]

About this brochure

You have been given this brochure because your child has been prescribed vosoritide.

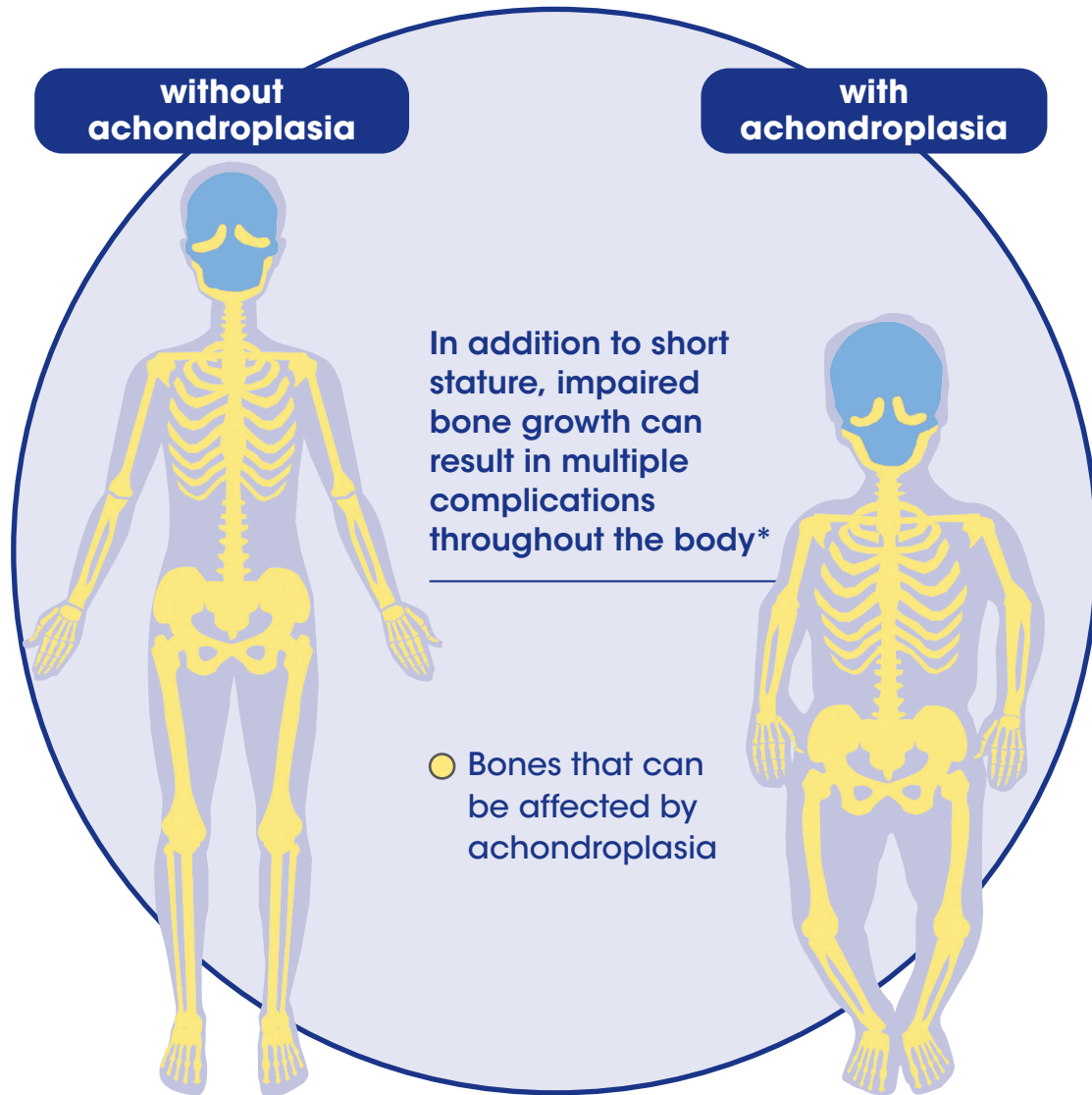
This brochure will give you and your family an overview of achondroplasia and how vosoritide can help to improve bone growth in children 4 months of age and older whose bones are still growing. Although this brochure contains information about your child's treatment, **it's important to follow the advice of your child's doctor and to ask them if you have any questions.**

Table of contents

What is achondroplasia?	3
How does vosoritide work?	5
What are the possible side effects of vosoritide?	6
How do I give a vosoritide injection?	7
Frequently asked questions	8
Working with your healthcare team	9
Introducing your child to therapy	10

What is achondroplasia?

Achondroplasia slows growth in almost all bones in the body



The majority of bones begin as cartilage before the body changes them into bone



These bones start to grow before birth and continue for as long as the bones' growth plates stay open (usually until young adulthood)

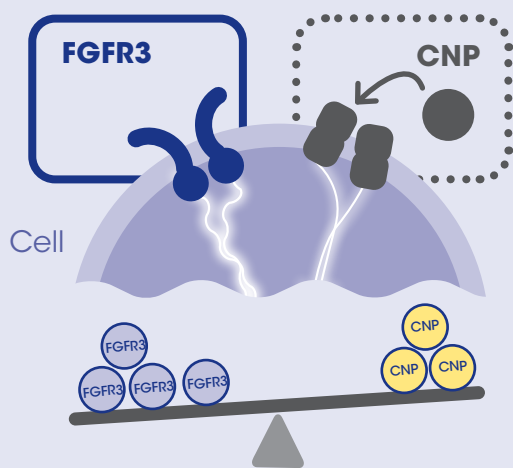


The balance between the activities of fibroblast growth receptor 3 (FGFR3) and C-type natriuretic peptide (CNP) is important for bone growth

*Vosoritide is approved for the treatment of achondroplasia in children 4 months of age and older whose bones are still growing; however, it has not yet been shown to have an effect on other complications.

What is achondroplasia?

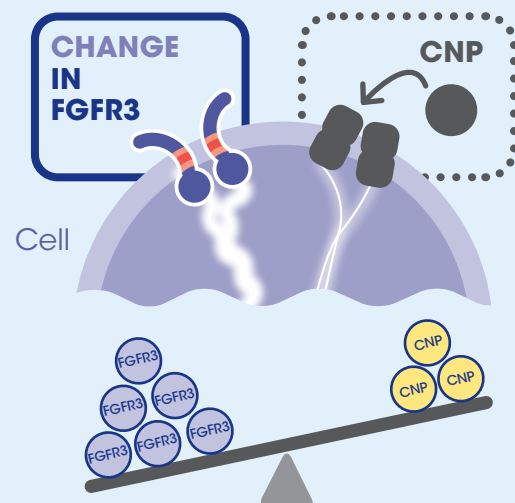
In people with achondroplasia, a change (mutation) in a gene called *FGFR3* stops bones from growing how they should. The *FGFR3* gene provides instructions for making the FGFR3 protein, which along with other proteins in the body like CNP, controls signals that tell the bones when to keep growing and when to stop. In people with achondroplasia the mutated gene causes FGFR3 to be overactive, which interferes with bone growth. Here's how:



Bone development without achondroplasia

In growth plates, a balance between FGFR3 and CNP signaling pathways occurs within the cells. FGFR3 slows bone growth while CNP promotes bone growth

FGFR3 and CNP signaling pathways do a balancing act until bones are done growing



Bone development with achondroplasia

With achondroplasia, FGFR3 sends more signals to slow bone growth than it should

The body's CNP is unable to balance overactive FGFR3 signaling

This imbalance affects the conversion of cartilage into bone, especially in the long bones of the arms and legs. This means that people with achondroplasia have short stature, with particularly short upper arms and thighs. It can also cause problems in other parts of the body.

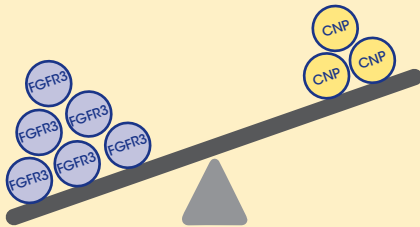
To learn more about achondroplasia, please visit achondroplasia.com

How does vosoritide work?

Vosoritide is a prescription medicine that is used to improve bone growth by helping CNP balance FGFR3 in children 4 months of age and older whose bones are still growing. It is only approved for achondroplasia caused by mutations in the *FGFR3* gene that have been confirmed by genetic testing.

The active ingredient in vosoritide is a modified form of the CNP the body produces naturally.

In achondroplasia, the body's CNP signaling pathway is unable to counteract too much FGFR3 signaling

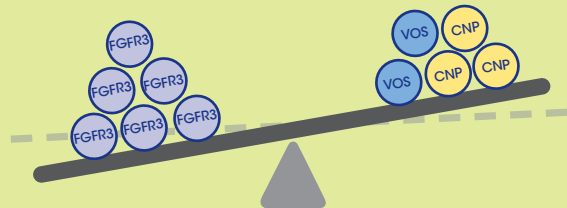


The body's CNP signaling pathway works to promote bone growth by counteracting FGFR3 signaling



Vosoritide is a CNP analogue, so it can work alongside the body's CNP

Vosoritide bolsters the body's CNP signaling pathway



Vosoritide

--- without achondroplasia

— with achondroplasia and vosoritide

1. FGFR3 sends more signals to tell bones to slow down growth
2. Vosoritide, a CNP analogue, targets the underlying cause of achondroplasia by helping to counteract overactive FGFR3 signaling

This graphic shows how vosoritide works but is not to scale and doesn't reflect a percentage of growth. Because growth rates for children with achondroplasia vary over time and by individual, the impact of vosoritide does as well.

Vosoritide helps to improve bone growth in achondroplasia in children 4 months of age and older whose bones are still growing.

What are the possible side effects of vosoritide?

Like all medicines, vosoritide can cause side effects, although not everyone experiences them.

Very common side effects (may affect more than 1 in 10 people):

- Vomiting
- Low blood pressure
- Injection site reactions: redness, itching, inflammation, swelling, bruising, rash, hives, pain. Injection site reactions are usually minor and resolve on their own within a few hours

Vosoritide can lower blood pressure. Temporary effects include dizziness, feeling tired or feeling sick shortly after an injection. If these effects are severe, tell your child's doctor.



Dizziness



Tiredness



Feeling sick

Please see the package leaflet for a full list of side effects. If your child gets any side effects, talk to your child's doctor, a pharmacist or nurse. This includes any possible side effects not listed in the package leaflet. Reporting side effects can help provide more information on the safety of this medicine.

How do I give a vosoritide injection?

Vosoritide is a once-daily injection under the skin that works directly on the growth plates of the bones to improve bone growth.

To get the most out of vosoritide therapy, you will be trained by a healthcare specialist on how to give your child daily injections.



Doctor checkups will monitor growth every **3 to 6 months**



The doctor can **adjust the vosoritide dose, if needed**

There are five steps to preparing and injecting vosoritide:



1 Prepare your supplies



2 Prepare the vosoritide injection



3 Select and clean the injection site



4 Give the **injection** (remember: PINCH, RELEASE, PUSH)



5 Clean up

To support your training, you will also receive a caregiver dosing and administration guide and video (scan QR code below to access) that provide step-by-step information on how to prepare and administer the vosoritide injection. If you do not have these guides, please ask your child's care team.

If you have any further questions on the use of this medicine, ask your child's doctor. Please see the accompanying package leaflet.



Scan with your phone or visit vosoritideinjection.com to watch how to inject vosoritide.

For more detailed advice, refer to the package leaflet or speak to your child's doctor, nurse or pharmacist.

Frequently asked questions

Q&A

How long will my child need to take vosoritide?

Vosoritide is given once daily as long as your child's growth plates are still open – you should continue to give your child vosoritide until your child's doctor tells you to stop. Regular checkups with your child's doctor will help to determine when their growth potential has been reached.

What should I do if I give more vosoritide than I should?

If you inject more vosoritide than you should, contact your child's doctor straight away.

What should I do if I forget to give vosoritide?

If your child misses a dose, the injection should still be given if it is within 12 hours of the scheduled time. If more than 12 hours have passed since the scheduled dose time, do not inject the missed dose. Wait until the next day and continue with the usual dose at the usual time.

What should I do if I stop giving vosoritide?

Always talk to your child's doctor before deciding to stop your child's treatment. If you have any further questions on the use of this medicine, ask your child's doctor or pharmacist.

Does vosoritide interact with any other medication?

Because vosoritide is a recombinant human protein, it is unlikely to cause drug interactions. Tell your child's doctor if your child is taking, has recently taken, or might take any other medicines.

What should I do if my child gets any side effects?

If your child gets any side effects, talk to your child's doctor, a pharmacist or nurse.

More questions? Bring them with you to the next appointment at your child's clinic



Working with your healthcare team

Use this page to track your child’s care team, as well as any other important information related to their care

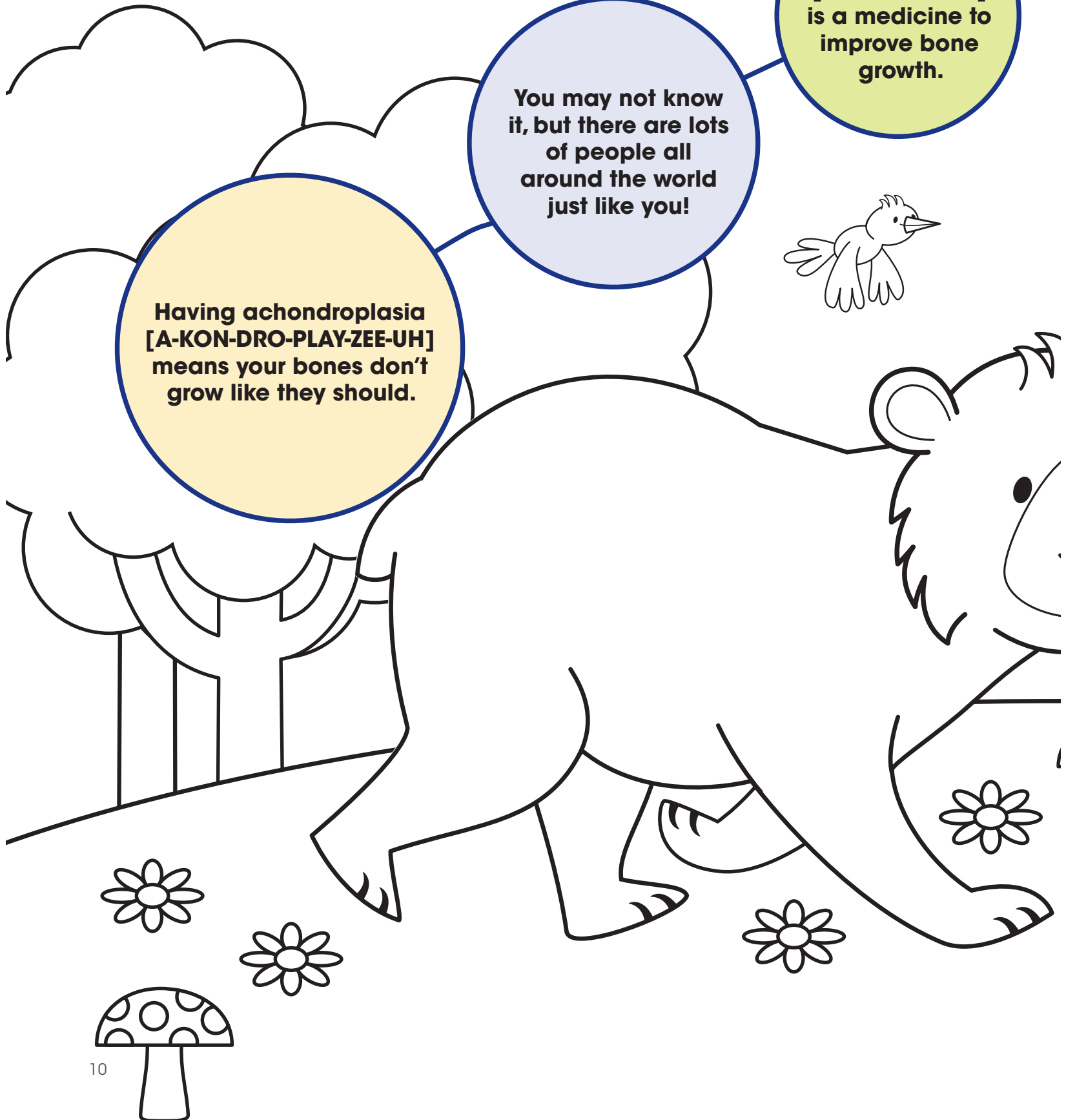
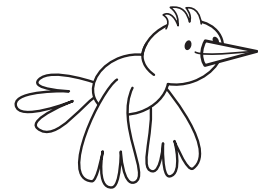
Introducing your child to therapy





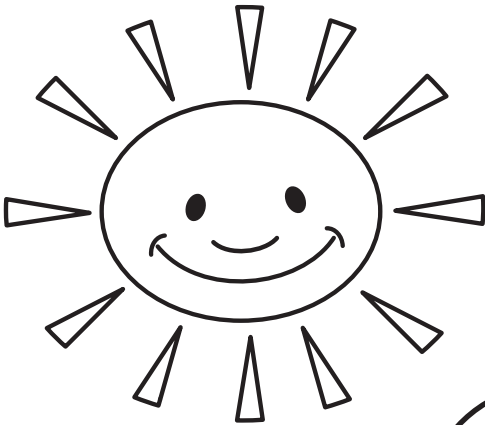

Because vosoritide must be given every day, your child will need to get used to daily injections. This page can be used to help incorporate vosoritide into your child's daily routine in a fun and interactive way.

**Vosoritide
[VO-SOR-E-TYD]
is a medicine to
improve bone
growth.**

**You may not know
it, but there are lots
of people all
around the world
just like you!**

**Having achondroplasia
[A-KON-DRO-PLAY-ZEE-UH]
means your bones don't
grow like they should.**





The medicine is injected into your arm, leg or tummy.

It's important to have your injection every single day while you are growing up. Soon it will be a normal part of your day, just like brushing your teeth.

You can help by being brave and strong, just like Big Bear!

To learn more about achondroplasia, visit achondroplasia.com



Scan with your phone or visit vosoritideinjection.com to learn more about how to inject vosoritide.

B:OMARIN[®]

Developed and Funded by BioMarin International Ltd.
© 2024 BioMarin International Ltd. All rights reserved.
EUCAN-VOX-00239 April 2024