

Burosumab treatment for X-linked hypophosphataemia (XLH)

Information for children, families and carers

We have given you this factsheet because your child's doctor has recommended that they have a new type of treatment for their X-linked hypophosphataemia (XLH) called burosumab. It explains what burosumab is, what the treatment involves and what the potential benefits and risks are. We hope it will help to answer some of the questions you may have. If you have any further questions or concerns, please speak to a member of our team.

What is burosumab?

Burosumab is a new type of treatment for the rare form of rickets called X-linked hypophosphataemia (XLH). XLH is a genetic condition that affects bones, muscles and teeth.

How does the treatment work?

Children with the condition XLH have too much of a hormone called fibroblast growth factor 23 (FGF23) in their blood. This hormone increases the amount of phosphate (a mineral that is essential for the normal growth of healthy bones and teeth) that is removed from the blood by the kidneys. As a result, the amount of phosphate in the blood of a child with XLH is lower than it should be. Without enough phosphate in their blood, a child's bones will not develop properly and will be too soft to support their own body weight without bending (bowed legs).

Burosumab is a medication that reduces the effect of the hormone FGF23 on the kidneys. This helps children with XLH to maintain a normal level of phosphate in their blood.

What are the benefits of this treatment?

In children, burosumab has been shown to:

- prevent irreversible bone damage
- help with healthy bone growth
- reduce bone pain
- improve quality of life (for example, being able to walk faster or play for longer)

Another benefit of burosumab is that your child will be able to stop taking their daily phosphate, calcium and activated vitamin D (such as calcitriol or alfacalcidol) medications and just have one injection every two weeks.

How should I prepare for my child's treatment?

Medications

Your child must stop taking their phosphate, calcium and activated vitamin D (such as calcitriol or alfacalcidol) medications one week before their first burosumab injection. They will not need to take these medications again while they are having their burosumab injections. They should continue their regular vitamin D supplements, such as colecalciferol.

Blood tests

To start with, your child will need to have a blood test before each burosumab injection. The blood test will be done on the same day as your child's injection. The blood tests will be arranged by the children's endocrine team. They will send you a letter in the post with the times and dates of each blood test.

The blood tests will help us to work out what dose of burosumab your child needs. Once we have decided on a suitable dose, your child will not need to have as many blood tests. We will contact you in advance to arrange future blood tests.

Eating and drinking

From midnight on the appointment days where a blood test is needed, your child must not eat or drink anything except water (this is called fasting). We recommend bringing some food with you to your child's appointment, so they can eat straight after their blood test.

What does the treatment involve?

We will give your child a subcutaneous (just under the skin) injection of burosumab every two weeks into their upper leg or buttocks. If needed, we can apply a numbing cream or a cold spray to the area before injecting the medication to make this as comfortable as possible for your child.

We will give your child their first seven burosumab injections at Southampton General Hospital in our children's outpatient department or on John Atwell day ward. We will send your child an appointment letter in the post which will confirm where you need to go for your child's appointment.

After the first injection, we may ask your child to stay in hospital for up to an hour. This is so we can make sure they do not have a reaction to the medication. If your child does not have a reaction to the medication, they will be able to go home immediately after having the following six injections in hospital.

After the first seven injections in hospital, a nurse working for healthcare company Alcura will give the burosumab injections to your child at your home. We will give you more information about this over the next few weeks.

Are there any risks or side effects?

The most common side effects of burosumab include:

- redness, pain, or swelling at the injection site
- a tooth abscess (infection)
- a cough
- headaches
- nausea (feeling sick) or diarrhoea
- muscle pain
- a fever

If you have any concerns about side effects your child is experiencing, please contact us.

Will my child need any follow-up care?

Most children will need to have burosumab treatment until they finish growing. This is usually around the age of 18.

Contact us

If you have any questions or concerns, or if any of your child's appointment times are not suitable, please contact us as soon as possible.

Children's endocrine clinical nurse specialists

Telephone: **023 8120 8719**

Email: **pens@uhs.nhs.uk**

Children's endocrine secretary

Telephone: **023 8120 6985**

Useful links

www.xlhuk.org

Hospital visits

We have included the table below to help you keep track of your child's appointments. Please make a note of any side effects your child experiences after having the burosumab treatment.

Hospital appointments	Date	Is a fasting blood test needed?	Your child's weight (kg)	Burosumab dose and injection site	Comments (Please record any side effects your child experiences)
1		Yes			
2		Yes			
3		Yes			
4		No			
5		Yes			
6		No			
7		Yes			

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