Frequently Asked Questions

What is the new treatment, and how does it work?

The new treatment is known as burosumab and marketed as Crysvita®. It is the first treatment that specifically targets the mechanism of hypophosphatemia in X-Linked Hypophosphatemia (XLH), which relates to the excessive levels of a hormone known as FGF23. Burosumab is a fully human monoclonal antibody that binds to FGF23, inhibiting its phosphate-wasting action in the kidneys. The treatment consists of a subcutaneous injection to be administered by a health professional, once every two weeks for children and once every four weeks for adults. Dosage is based on weight, and blood tests should be monitored to make sure dosage is optimal, generally more frequently at the beginning of treatment than after adjustments in dose are individualized. Burosumab is a treatment, not a cure, so stopping the injections will result in a return to low phosphorus levels.

Is burosumab right for me or my child?

The approval of burosumab by the Food and Drug Administration (FDA) in the U.S., and by the European Medicines Agency in Europe, means that it has been reviewed by them and found to be both safe and effective for treatment of XLH. In the U.S., burosumab has been approved for both pediatric and adult XLH patients, while in Europe, it has been approved only for pediatric and young-adult use at this time. It is not currently approved for other forms of hypophosphatemia, such as autosomal hypophosphatemia or tumor-induced osteomalacia, but may be in the future. In any event, you will need to speak with your own physician to determine if this treatment is right for you or your child.

How can burosumab help me?

Every patient is different, so there is no way to say specifically the impact burosumab will have on any particular person's symptoms. The results of the clinical trials of burosumab appear to indicate a definite improvement in areas that patients find the most troublesome. Pain and the use of pain medication decreased over the course of initial clinical trials, while stiffness and joint function improved and fractures healed more quickly. Benefits were apparent in both adults and children, who also had improvement in their bone growth. While these are all encouraging results, only you and your physician can determine if burosumab is right for you.

Will it address dental issues such as abscesses?

Dental issues were not followed during the clinical trials, so we do not yet know the impact of burosumab on the teeth. However, dental issues will be among the topics that the Network will be following with its Natural History Study over a ten-year period, seeking a better understanding of how this new treatment affects all aspects of a patient's health.

Are there any safety issues or concerning side-effects?

No life-threatening or major adverse side-effects have been reported in the clinical trials. Some minor side-effects have been reported, such as injection site reactions or other allergic reactions, and events that may or may not have been related to the treatment, such as headache, dizziness, diarrhea, cramps, indigestion, nausea, and heartburn. No new kidney calcification has been observed, and parathyroid hormone levels have not worsened. However, burosumab has been studied in only a relatively small number of patients for a relatively short period of time, so additional studies will be necessary to determine whether there are additional adverse side-effects with long-term use.

Will burosumab replace my current treatment?

During clinical trials, patients discontinued their phosphorus and calcitriol supplements, and their phosphorus levels normalized on burosumab alone. However, only you and your doctor can determine the best treatment plan for you. It is contraindicated to give phosphorus and calcitriol in usual therapeutic dosages for XLH when burosumab is being used.

How soon will burosumab be available in the United States?

From the date of approval by the FDA, we expect that it will take about six months for burosumab to be widely available and for insurance companies to begin to be educated on the importance of this new treatment. Ultragenyx Pharmaceutical is the company responsible for distribution of burosumab in the United States, and they have created a program called Ultracare to help patients through the process of dealing with insurance companies and gaining access to the medication. For more information about Ultracare, call 1-888-756-8657 or visit www.ultracaresupport.com.

What about those living outside of the United States? What is the time frame for approval in my country?

The European Medicines Agency has approved burosumab for the treatment of XLH in children one year of age and older and adolescents with growing skeletons. There are additional steps that individual European countries must follow in order to ensure access, and the Network will share that information as it is available. We do not currently have information on countries outside of the U.S. and Europe, but will be working to find out more. The latest updates will be posted on our website at www.xlhnetwork.org and on our social media platforms. There are also local organizations forming in a number of countries, and The Network is happy to put patients who live outside the U.S. in touch with them; simply email us for more information at info@xlhnetwork.org.

What happens to patients who are currently receiving burosumab as part of the clinical trials? How do they transition to a local clinician?

When the clinical trials end, patients participating in them will, if they were not already seeing the trial's clinician, need to find a specialist near where they live. The new clinician will be able to obtain sufficient information from the trial's principal investigator to understand the patient's treatment during the trial. For assistance in finding a local clinician, the Network may be able to provide a name of a local clinician who has XLH experience. Just contact us at info@xlhnetwork.org.

Who can prescribe burosumab?

If burosumab is determined to be an appropriate treatment, any licensed physician will be able to write a prescription for it just like for any other pharmaceutical product. We recommend, however, that you seek treatment from a specialist, someone who is both trained to deal with metabolic disorders and has experience with XLH. For assistance in finding a local clinician, the Network may be able to provide the name of a local clinician who has XLH experience. Just contact us at info@xlhnetwork.org.

Will I be able to afford burosumab?

In the United States, health care is expensive, and that includes pharmaceutical treatments. The Network is advocating for all patients to have access to the best treatment possible and will be sharing as many resources as it can find to make treatment affordable. Ultragenyx Pharmaceutical has assured us that they are working toward a goal of ensuring that everyone who needs burosumab will be able to receive it, regardless of their financial circumstances. We are working on a guide to patient assistance programs that can help with insurance deductibles and copays and will share it at our website and social media platforms.

How much will my insurance cover?

Coverage depends on two factors. First, the amount of your drug coverage co-pay, as set out in your insurance plan. You can find that in your policy documents, along with any related issues, such as deductibles applicable to drug coverage. Second, the amount your insurance will cover depends on whether burosumab is listed in its formulary as a covered treatment. There will be approximately a six-month period after the FDA's approval of burosumab when it will not yet be in the formulary, while the insurance companies are reviewing burosumab and determining whether it will be included in the formulary. During this time, conditional approval *may* be given, but is not required.

Where can my physician get more information about burosumab?

Below is a recent article your physician may find helpful in learning about this new treatment. You can also access numerous articles related to treatment of XLH at the Network's forum: www.forum.xlhnetwork.org.

"X-linked hypophosphatemia and FGF23-related hypophosphatemic diseases—Prospect for new treatment." *Endocrine Review*, January 2018 https://www.ncbi.nlm.nih.gov/pubmed/29381780