Crysvita (burosumab)
An overview of Crysvita and why it is authorised in the EU

What is Crysvita and what is it used for?

Crysvita is a medicine used for the treatment of X-linked hypophosphataemia, a hereditary disorder characterised by low levels of phosphate in the blood (hypophosphataemia). Phosphate is essential to build bones and teeth and to maintain their strength, so patients may develop rickets and other bone deformities and growth problems.

Crysvita can be used in children and adolescents between 1 and 17 years of age when signs of bone disease are seen on X-rays, and in adults.

Crysvita contains the active substance burosumab.

X-linked hypophosphataemia is rare, and Crysvita was designated an ‘orphan medicine’ (a medicine used in rare diseases) on 15 October 2014. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu3141351.

How is Crysvita used?

The medicine can only be obtained with a prescription and treatment should be started by a doctor experienced in the management of patients with bone diseases caused by alterations in the body’s chemical processes.

Crysvita is given as an injection under the skin. The recommended starting dose in children and adolescents up to 17 years of age is 0.8 mg per kg of body weight every two weeks. The maximum dose is 90 mg every two weeks. From the age of 18, the recommended starting dose is 1 mg per kg of body weight, up to a maximum dose of 90 mg every four weeks. The dose is adjusted according to the patient’s phosphate levels in the blood.

For further information about using Crysvita, see the package leaflet or contact your doctor or pharmacist.

How does Crysvita work?

Phosphate levels are largely controlled by the kidneys, which either remove excess phosphate or reabsorb it into the bloodstream when needed. Patients with X-linked hypophosphataemia have
abnormally high levels of a protein called FGF23 which causes the kidneys to stop reabsorbing phosphate into the bloodstream.

Crysvita is a monoclonal antibody (a type of protein) designed to recognise and attach to the FGF23 protein. By attaching to the FGF23 protein, the medicine blocks its activity, allowing the kidneys to reabsorb phosphate and restore normal levels of phosphate in the blood.

**What benefits of Crysvita have been shown in studies?**

Crysvita reduced the severity of rickets as shown in X-rays in patients with X-linked hypophosphataemia.

The medicine was assessed in one main study in 52 children aged between 5 and 12 years. All the children received Crysvita either every two weeks or every four weeks. The main measure of effectiveness was a reduction in the severity of rickets (bone deformities) in the wrist and knee measured on a scale from 0 (normal) to 10 (severe). The average score before treatment was 1.9 points in children given Crysvita every 2 weeks, and this fell by 1.0 point after 64 weeks of treatment; in those given the medicine every 4 weeks the baseline score of 1.7 fell by 0.8 point. In addition, phosphate levels in the blood improved over time in both groups, particularly those given Crysvita every 2 weeks. A study in 13 younger children shows that Crysvita is effective in those aged between 1 and 4 years.

Another study investigated the use of Crysvita in 134 adults. Patients were given Crysvita or placebo (a dummy treatment) every four weeks for 24 weeks, and the main measure of effectiveness was the normalisation of phosphate levels in the blood. The study showed that blood phosphate levels returned to normal values in 94% of patients given Crysvita, compared with 8% of patients on placebo.

**What are the risks associated with Crysvita?**

In children, the most common side effects with Crysvita (which may affect more than 1 in 10 people) are injection site reactions (such as skin redness, itching, rash, pain and bruising), cough, headache, fever, pain in arms and legs, vomiting, tooth abscess, decreased vitamin D level, diarrhoea, rash, nausea (feeling sick), constipation, dental caries and muscle pain.

In adults, the most common side effects (which may affect more than 1 in 10 people) are back pain, headache, tooth infection, restless legs syndrome, muscle spasms, decreased vitamin D level and dizziness.

Oral phosphate and active forms of vitamin D (such as calcitriol) must not be used during treatment with Crysvita. Also, Crysvita must not be used in patients with high phosphate levels in the blood or in patients with severe kidney disease.

For the full list of side effects and restrictions with Crysvita see the package leaflet.

**Why is Crysvita authorised in the EU?**

Crysvita was shown to reduce the severity of bone deformities in the wrist and knee and improve the level of phosphate in the blood of children with X-linked hypophosphataemia in one study, and further data are awaited. The medicine was also effective at improving phosphate blood levels in adults. Its side effects seem manageable. The European Medicines Agency therefore decided that Crysvita’s benefits are greater than its risks and recommended that it be approved for use in the EU.
Crysvita has been given ‘conditional authorisation’. This means that there is more evidence to come about the medicine, which the company is required to provide. Every year, the European Medicines Agency will review any new information that becomes available and this overview will be updated as necessary.

**What information is still awaited for Crysvita?**

Since Crysvita has been granted a conditional approval, the company that markets Crysvita will provide updated results from a study in children aged between 1 and 4 years.

**What measures are being taken to ensure the safe and effective use of Crysvita?**

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Crysvita have been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Crysvita are continuously monitored. Side effects reported with Crysvita are carefully evaluated and any necessary action taken to protect patients.

**Other information about Crysvita**

Crysvita received a conditional marketing authorisation valid throughout the EU on 19 February 2018.

Further information on Crysvita can be found on the Agency’s website:  

This overview was last updated in 10-2020.