National Centre for Pharmacoeconomics
NCPE Ireland

NCPE Plain English Summary

Drug name: Burosumab for the treatment of X-linked hypophosphataemia (XLH) with radiographic evidence of bone disease in children one year of age and older and adolescents with growing skeletons. **Brand name: Crysvita®**

What is the NCPE?

The National Centre for Pharmacoeconomics (NCPE) is a team of experts who look at the health benefits and costs of medicines. The HSE asks us to advise on whether or not a new medicine is good value for money. We give unbiased advice to help the HSE provide the most effective, safe and cost-effective (value for money) treatments for patients.

How do we make our recommendations?

Our main focus is on the health benefits and cost effectiveness of a medicine. We look at the wider costs and health benefits associated with a new medicine, for example:

- Does the new medicine work better than other treatments available in Ireland?
- Is the new medicine easier to give or easier to take compared with other treatments available in Ireland?
- Does the new medicine reduce the need for patients to be hospitalised?
- Does the new medicine improve the quality of a patient's life over other treatments available in Ireland?
- Will the new medicine save resources elsewhere within the health system?

We review the information from clinical trials along with the cost and value for money data presented by the pharmaceutical company. We ask doctors and other healthcare professionals for advice about any health benefits of the new medicine compared with current treatments. We also ask patient organisations to send us their views on how the new drug may improve patients' day-to-day experience of living with a disease.

What is Burosumab used for?

X-linked hypophosphataemia (XLH) is a chronic, progressive, debilitating multisystem disease. It is characterised by low levels of phosphate in the blood (hypophosphataemia). Phosphate is essential to build bones and teeth and to maintain their strength. Affected patients have skeletal abnormalities and the main clinical consequence in children is rickets. When these deformities become permanent, people with XLH suffer lifelong disability and pain. The long term goal of therapy in children with XLH is to improve or heal rickets and prevent or correct the skeletal abnormalities associated with it. People with XLH have abnormally high levels of a protein (called FGF23) that stops the kidneys reabsorbing phosphate into the blood. Burosumab works by blocking the FGF23 protein, which allows the kidneys to reabsorb phosphate. This raises the phosphate levels in the blood, reducing the risk of bone defects in children**.**

What recommendation has the NCPE made to the HSE?

We have recommended that the HSE should consider not funding burosumab unless its cost effectiveness (value for money) can be improved. The HSE will consider our recommendation and make the final decision about reimbursement (funding). When making the funding decision, the HSE will also consider the additional criteria outlined in the Health (Pricing and Supply of Medical Goods) Act 2013.

Why did we make this recommendation?

After reviewing the data presented by the pharmaceutical company, we concluded that burosumab may work as well or better than other ways to manage this condition. However, the price of burosumab is too high compared to how the condition is currently managed, and therefore we believe that the medicine is very poor value for money. We recommend that the HSE consider **not** providing this medicine unless the HSE can agree a suitable price reduction with the pharmaceutical company.

Next steps

When the HSE receives our recommendation, it will look at all the relevant data about burosumab. The HSE makes the final decision on reimbursement.

Where can I get more information?

You can get more information about burosumab from the following online options:

- the NCPE Technical Summary Document
- Crysvita® European Public Assessment Report (EPAR) – Summary for the public

  or
- searching for burosumab on our website (www.ncpe.ie);
- searching for burosumab on the European Medicines Agency (EMA) website (www.ema.europa.eu).

Please refer to the NCPE website for updated information on the reimbursement status of this medicine.

Date published: **March 2020**