Assessment explained

Medicine: burosumab (brand name: Crysvita®) for X-linked hypophosphataemia in children and adolescents

Kyowa Kirin Ltd

Burosumab meets the SMC definition of an ultra-orphan medicine, which is a medicine used to treat an extremely rare condition. This document summarises the initial SMC assessment of burosumab.

What does this mean for patients?

If your healthcare professional thinks that burosumab is the right medicine for you, you should be able to have the treatment on the NHS in Scotland within the **ultra-orphan pathway** (see next page). This is provided the company submits a plan to the Scottish Government describing how further data, including on the patient and carer lived experience, will be collected over the next 3 years. After this, SMC will reassess the medicine and make a decision on routine availability.

What is burosumab used for?

Burosumab is used to treat X-linked hypophosphataemia (XLH), which is a very rare inherited condition that causes low levels of phosphate in the blood. Phosphate is very important in building bones and teeth and keeping them strong. In XLH, low levels of phosphate can result in painful skeletal deformities (such as rickets) which develop in childhood and persist into adulthood. The condition can have a profound effect on day to day functioning and quality of life.

Burosumab is for children who are 1 year of age or older and adolescents whose bones are growing.

How does burosumab work?

The kidneys play an important role in controlling the level of phosphate in the bloodstream. 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.

How do we assess ultra-orphan medicines?

SMC uses a broad assessment framework for ultra-orphan medicines. This is part of a new ultra-orphan pathway in NHSScotland which has **four stages**:

Stage 1 Validation

SMC reviews information from the company to decide if the ultra-orphan definition is met

The company must then agree to further requirements of the ultra-orphan pathway*.

Stage 2 Initial Assessment

SMC considers the:

- nature of the condition
- health benefits of the medicine
- impact beyond direct health benefits and on specialist services
- value for money, and
- costs to the NHS.

Stage 3 Evidence Generation

The medicine can be prescribed while the company gathers futher data, including on the patient and carer lived experience.

Stage 4 Reassessment

After 3 years the company provides an updated submission for reassessment.

SMC considers all the evidence and makes a decision on routine use of the medicine in NHSScotland.

*provide a confidential discount known as a Patient Access Scheme (PAS) to increase the cost-effectiveness of the medicine, and provide a data collection plan.

What have we said in this assessment?

- Clinical studies in children aged 1 to 12 years show that burosumab improves rickets compared with current treatment. However currently it is unclear how this medicine affects progression of the disease into adulthood and how this impacts patients' quality of life.
- Despite the confidential discount (PAS) offered by the company, the cost in relation to the health benefits of burosumab remains high.

For further information please see the SMC ultra-orphan medicine initial assessment report (SMC2240).

More information

The organisations below can provide more information and support for people with X-linked hypophosphataemia and their families. SMC is not responsible for the content of any information provided by external organisations.

Metabolic Support UK

https://www.metabolicsupportuk.org/

0845 241 2173

XLH UK

https://xlhuk.org/

You can find out more about burosumab in the European public assessment report (EPAR) summary for the public by searching for the medicine name on the European Medicines Agency (EMA) website.

www.ema.europa.eu

Date assessment report published: 10 February 2020 **SMC No: SMC2240**