15 December 2017
EMA/CHMP/824779/2017
Media and Public Relations

Press release

New medicine for rare bone disease

Crysvita, a medicine for the treatment of X-linked hypophosphataemia, recommended for conditional approval

The European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) has recommended granting a conditional marketing authorisation in the European Union for Crysvita (burosumab), a medicine for the treatment of X-linked hypophosphataemia (XLH) with radiographic evidence of bone disease in children 1 year of age and older and adolescents with growing skeletons.

XLH is an inherited disorder characterized by low levels of phosphate in the blood. The phosphate is abnormally processed in the kidneys, which causes a loss of phosphate in the urine (phosphate wasting) and leads to soft, weak bones (rickets). In most cases, the signs and symptoms of hereditary hypophosphataemic rickets begin in early childhood. Characteristic features include bowed or bent legs, short stature, bone pain, and severe dental pain.

The CHMP recommended conditional approval for the medicine. This is one of EU's regulatory mechanisms to facilitate early access to medicines that fulfil unmet medical need. Conditional approval allows the Agency to recommend a medicine for marketing authorisation in the interest of public health where the benefit of its immediate availability to patients outweighs the risk inherent in the fact that additional data are still required.

There is currently no authorised medicine available to treat this rare, serious, chronic and debilitating disease. Most children with XLH receive conventional therapy consisting of multiple daily doses of oral phosphate and active vitamin D analogues. The benefits of Crysvita are its ability to reduce the loss of phosphate, improve abnormally low serum phosphate concentrations and other metabolic changes, and to reduce the severity of rickets as shown in x-rays.

The CHMP's recommendation is based on two phase II studies. The main study was conducted on 53 children aged 5-12 years. Children treated with Crysvita experienced an improvement in their phosphate level and in the reabsorption of phosphate in their kidneys as well as radiographic improvement of rickets. In the second study of 13 patients age 1 to 4 years old receiving Crysvita, the response was similar than in children in the main study. On this basis, the CHMP considered that efficacy results from age group 5-12 years can be extrapolated to ages 1 to 4 years old. As part of the conditional marketing authorisation, the applicant is required to complete three ongoing studies to

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 8427 **Facsimile** +44 (0)20 3660 5555
E-mail press@ema.europa.eu **Website** www.ema.europa.eu

further investigate the safety and efficacy of the medicine. The data from all three studies are planned to be submitted in or before 2020.

The most common adverse reactions observed with Crysvita were injection site reactions, headache, and pain in extremities.

The CHMP recommended that Crysvita will be prescribed by physicians experienced in the management of patients with metabolic bone diseases.

Because XLH is rare, Crysvita received an orphan designation from the Committee for Orphan Medicinal Products (COMP) in October 2015. As always at time of approval, this orphan designation will now be reviewed to determine whether the information available to date allows maintaining burosumab's orphan status and granting this medicine ten years of market exclusivity.

The opinion adopted by the CHMP at its December 2017 meeting is an intermediary step on Crysvita's path to patient access. The CHMP opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once a marketing authorisation has been granted, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role/use of this medicine in the context of the national health system of that country.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The Applicant for Crysvita is Kyowa Kirin Limited.
3. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

Contact our press officers

Tel. +44 (0)20 3660 8427
E-mail: press@ema.europa.eu
Follow us on Twitter @EMA_News