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PRESS RELEASE
For immediate distribution

Scottish Medicines Consortium confirms children's access to burosumab for treating X-linked hypophosphataemia (XLH)

London, England, 15th January 2024. XLH UK, and the community of patients and families that it represents, is pleased to report that the Scottish Medicines Consortium (SMC) has confirmed access to burosumab (marketed as Crysvita) for the treatment of X-linked hypophosphataemia (XLH) in children and adolescents aged 1 to 17 years with radiographic evidence of bone disease.

The recommendation for children was made following reassessment through the ultra-orphan framework, which is used for medicines for very rare diseases. The SMC took into consideration evidence of the effectiveness of burosumab and the views from a Patient and Clinician Engagement (PACE) meeting which XLH UK attended.

XLH is a rare genetic condition that if untreated can cause significant skeletal deformities in children from a young age, with lifelong disability, pain, and fatigue. It is a debilitating condition which can require patients to have multiple, corrective surgeries across the course of their lifetime. The news ensures Scottish children with XLH will continue to have access to the first and only treatment that targets the underlying mechanism of their hypophosphataemia.

We commend SMC's Ultra-Orphan pathway Committee, NHS Scotland and Kyowa Kirin International for their continued efforts to evaluate the impact that a rare, hereditary and lifelong disorder has on individuals and their families. We would like to thank all the patients and families who shared their experiences with us through our research using patient surveys, interviews and case studies. These helped us in preparing our patient organisation submission for the SMC, so that decision-makers could understand the impact of XLH on patients and their families.

More about XLH
More about burosumab
XLH UK
Kyowa Kirin International