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PRESS RELEASE
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Scottish Medicines Consortium enables access to burosumab for treating X-linked hypophosphataemia (XLH)

Adults with XLH will now be able to access burosumab on the NHS in Scotland.

London, England, February 13, 2023. XLH UK, and the community of patients and families that it represents, is looking forward to a brighter future now that the Scottish Medicines Consortium (SMC) has enabled access to burosumab (marketed as Crysvita) to treat adults who have a confirmed diagnosis of X-linked hypophosphataemia (XLH).

The recommendation for adults was made through the ultra-orphan pathway, which is used for medicines for very rare diseases. This pathway allows people with XLH to access burosumab while more data is collected to support its effectiveness. The SMC will review this evidence in three years to make a final decision on its routine use.

Scotland is the first of the four UK nations to recommend burosumab for adults with XLH. Before this decision, children over one year old and with growing skeletons could access burosumab though the ultra-orphan assessment in February 2020. Burosumab is also recommended for eligible children across all four UK nations.

XLH is a rare genetic condition inherited as an X-linked dominant trait 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 is truly life-changing for patients and their families with XLH as it is the first and only treatment that targets the underlying mechanism of their hypophosphataemia. This breakthrough therapy shows improvements in the areas that matter to patients, including reductions in daily pain and stiffness as well as improvements in healing of fractures which may limit the need for invasive, corrective surgeries and be without lifelong pain and disability.

Commenting on SMC's decision, XLH UK founder Oliver Gardiner explained that *"This is fantastic news for adults with XLH in Scotland and is a leap forward, setting a precedent as the first of the UK nations to recommend burosumab for the eligible adult population. This life-changing treatment is the first to tackle the underlying problem, with the potential to lessen the significant physical and emotional challenges that adults with XLH are faced with on a daily basis."*

We commend SMC's Ultra-Orphan pathway Committee, NHS Scotland and Kyowa Kirin International for their efforts, dedication and commitment to understand 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 immensely 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