

Strategic Review 2024 and Plan for 2025

Our mission is to help those with XLH and related disorders within the UK, through research, support and advocacy.

11th Jan 2025

Our Purpose

XLH UK exists to help those with XLH and related disorders within the UK, through research, support and advocacy.

Events

Submissions

News

We...

Listen

Represent

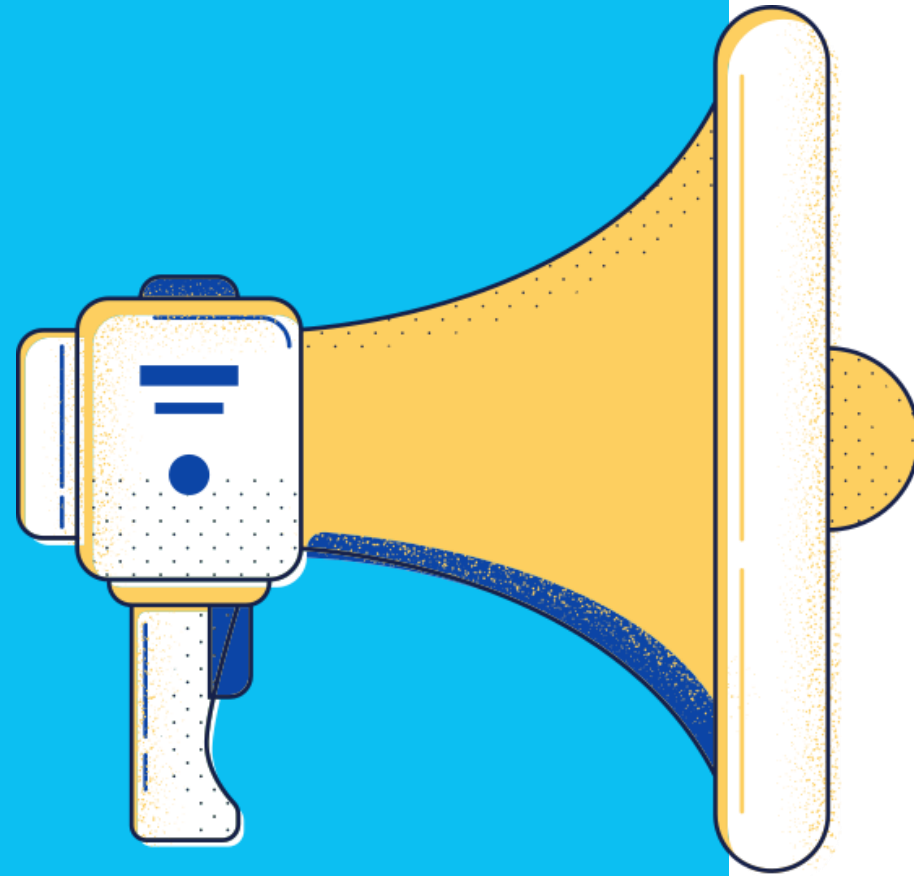
Inform

Research

Patient stories

Webinars

Our Strategic Goals



- Make a meaningful contribution to improving the quality of life of XLH patients and their families
- Build a high-performing sustainable organisation

Review of the year

A graphic of the year 2024. The '2' is purple, the '0' is a solid purple circle, the first '2' is a gradient from red to orange, and the '4' is a gradient from orange to yellow. The numbers are set within a white rounded rectangle with a thick cyan border.

2024

Achievements 2024

Advocacy

- Burosumab for adults with XLH: Agreed by NICE for England, Wales & NI
- New NHS guidelines for treating XLH adults, supported by patient leaflet

Research

- White paper - Exploring Perspectives of People with XLH
- Family & Carers survey

Support

- Community Day in Birmingham
- Connect online support meetings

Advocating for patients across UK

Northern Ireland
HTA

Scottish Medicines Consortium

NICE National Institute for Health and Care Excellence

Payer

Department of Health | An Roinn Sláinte
Mánnystrie O Poustie

Scotland
HTA

Scottish Medicines Consortium

Payer

NHS
SCOTLAND

Wales
HTA

All Wales Medicines Strategy Group
Grŵp Strategaeth Meddyginiaethau Cymru Gyfan

NICE National Institute for Health and Care Excellence

Payer

GIG CYMRU
NHS WALES

England
HTA

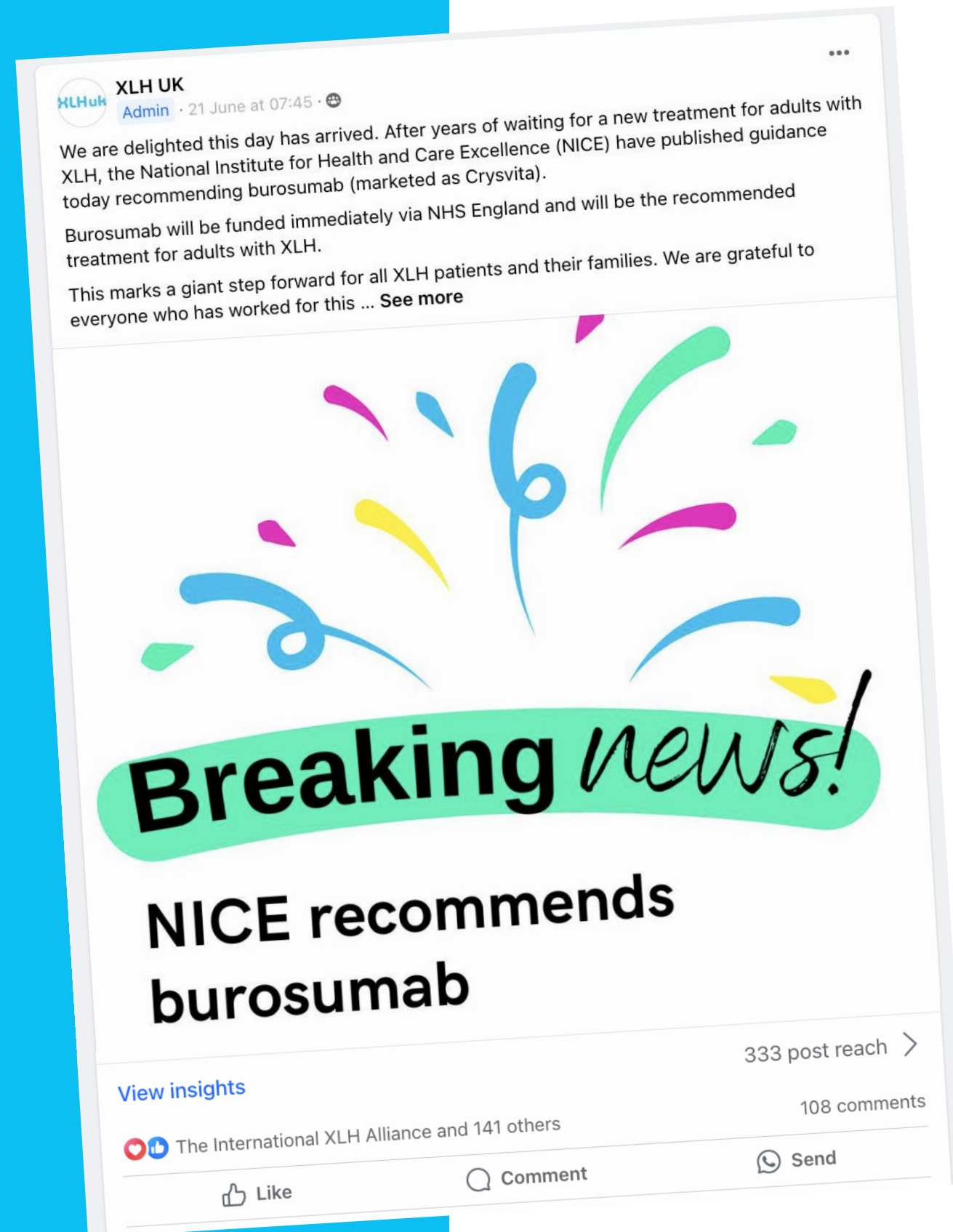
NICE National Institute for Health and Care Excellence

Payer

NHS
England



XLHuk.org



Special Webinars

NICE Guidance
explained

NHS Guidelines for adults with XLH

- XLH UK have produced a patient fact sheet in partnership with the clinicians
- This covers how to access and administer the treatment and some common questions
- It is available to download on the XLH UK website
<https://xlhuk.org/treatment/burosumab-and-xlh/adults-starting-on-burosumab/>

XLHuk

Drug treatments for Adults with X-Linked Hypophosphataemia: Burosumab (Crysvita®)

Burosumab is a new injectable drug treatment for children and adults with X-linked hypophosphataemia (XLH). It works to help the body keep the phosphate levels that it needs. This can help to prevent fractures, bone pain, fatigue, strengthen your bones, and may increase your mobility.

Who is this fact sheet for?
This information may be helpful if you (or someone close to you):

- have been diagnosed with X-linked hypophosphataemia (XLH) by a healthcare professional.
- have been advised to consider taking burosumab to help manage or prevent symptoms.

It includes the following information:

- What is XLH?
- What is burosumab?
- How does burosumab work?
- Who can have burosumab?
- How do you take burosumab?
- Getting the most out of your treatment.
- Getting more information and support.

What is XLH?
X-linked hypophosphataemia (XLH) is a rare genetic disorder that leads to low phosphate levels in the blood, which are crucial for healthy bones and teeth. XLH symptoms often show in early childhood, causing symptoms such as bowed legs, short stature, and delayed walking. As individuals with XLH age, they may experience bone pain, fractures, muscle weakness, and dental issues. The condition can vary widely, even among members of the same family. Early recognition and management are important to reduce the impact of these symptoms on daily life as they tend to get worse with age.

What is burosumab?
Burosumab (brand name Crysvita®) is a medication used in managing XLH and has been designed to treat low phosphate levels in the blood, which helps to improve bone health. It works by addressing the root cause of the phosphate imbalance, supporting normal bone development, and reducing symptoms like bone pain and muscle weakness.

How does burosumab work?
Burosumab is a targeted therapy designed to treat conditions caused by low phosphate levels in the blood. It works by blocking the activity of a protein called fibroblast growth factor 23 (FGF23). In normal physiology, FGF23 regulates phosphate levels in the blood by the kidneys, leading to increased phosphate excretion in the urine.

White Paper

- We commissioned Triangulate to analyse the evidence for burosumab improving adult patient's Quality of Life
- Using data from the XLH UK 2023 Patient survey
- It is available to download on the XLH UK website <https://xlhuk.org/research-library/xlh-library/>

XLHuk

**Exploring Perspectives of People with XLH:
Insights into Disease Impact, Daily Challenges,
and Treatment Views**

Family & Carers survey

- 116 patients and family members completed XLH UK's Carer Survey
- For adults with XLH on burosumab, the total average carer hours per week decreased by 61% since going on burosumab
- 78% of carers reported that the level of care they needed to provide had decreased

"I find it extremely hard to care for the person with XLH. Her health has deteriorated massively and she struggles to walk all the time. Doing everyday tasks is a hindrance for her and I have to be around all day just in case she struggles with her tasks or if she falls and hurts herself. It is affecting my mental health as well."

Community Day in Birmingham



Professor Kassim Javaid was keynote speaker

XLH-related talks



Community Day in Manchester



Fun for all the family



Connect for online support

- Bringing the value of shared experiences, through the introduction of Zoom meetings for the XLH community to meet virtually



Engaging the community

- Special webinars to inform patients of the timelines and implications of NICE guidance
- Support pack developed for fundraising with a Christmas raffle



Fundraising

- In 2024 we raised more than £2,000 in donations
- We used a grant from the National Lottery to fund the 2024 Community Day, including travel bursaries to allow more of the community to attend



Strategic Priorities 2025


1. Awareness & standards of care
2. Access to new treatments
3. Information and support
4. Research and evidence generation
5. High performing, sustainable organisation





Contact Information

How to reach out

-  <https://www.facebook.com/groups/xlhukcharity>
-  <https://www.linkedin.com/company/xlh-uk>
-  https://www.instagram.com/xlh_uk
-  <https://twitter.com/UkXlh>
-  contact@xlhuk.org
-  <https://xlhuk.org>