

News release

Kyowa Kirin Presents Real-World Evidence Demonstrating Clinically Meaningful Impact of Burosumab Treatment in Adults with X-linked Hypophosphatemia

- *Findings from UK burosumab early access program spotlighted in oral presentation at ASBMR annual meeting*
- *Statistically significant improvements seen in patient-reported measures of pain, stiffness, physical function, and health-related quality of life*

Galashiels and Marlow, United Kingdom., 1 October 2024 — Kyowa Kirin International (KKI), a wholly owned subsidiary of Kyowa Kirin Co., Ltd. (TSE:4151, Kyowa Kirin) and a Japan-based global specialty pharmaceutical company, presented new research showing statistically significant and clinically meaningful improvements in patient-reported outcomes (PROs) in adults with X-linked hypophosphatemia (XLH) following treatment with burosumab (CRYSVITA) in real-world clinical practice. These results were shared with attendees at the American Society for Bone and Mineral Research (ASBMR) 2024 annual meeting in Toronto, Canada, 27-30 September 2024.

“The improvements we observed in patient-reported symptoms and health-related quality of life provide additional evidence of the real-world impact of treatment on outcomes relevant to patients and clinicians,” said Judith Bubbear, MD, at the Royal National Orthopaedic Hospital in London, UK, and chief study investigator and author. “These benefits were accompanied by sustained improvements in serum phosphate concentration, the hallmark deficiency in people with XLH.”

XLH is a rare genetic, progressive, metabolic bone disease that causes skeletal abnormalities, stiffness, pain, and impaired physical function.¹ In this retrospective, longitudinal, real-world study, researchers assessed the experience of 136 burosumab-naïve adults with XLH enrolled in the UK burosumab early access program.

Investigators observed statistically significant ($p < 0.05$) and clinically relevant improvements in mean patient-reported outcomes after six and 12 months of burosumab treatment, including:

- Brief Pain Inventory-Short Form (BPI-SF) domains: Worst Pain, Pain Severity, and Pain Interference.
- Western Ontario and McMaster Universities Arthritis Index (WOMAC) for Stiffness, Physical Function, Pain, and Total Score.
- EuroQol 5-Dimension (EQ-5D) 5-Level utility and visual analogue scale scores, measures of health-related quality of life.

“XLH is a lifelong, progressive disease, and Kyowa Kirin is deeply committed to partnering with the clinical and XLH community to help advance our understanding of the disease and its impact on patients and families,” commented Ben Johnson, global HEOR lead for nephrology at Kyowa Kirin and study author. “These data enhance our understanding of the real-world effectiveness of burosumab treatment in adults with XLH, in terms of the impact on specific symptoms as well as general health-related quality of life.”

About X-linked hypophosphataemia (XLH)

XLH is caused by a genetic mutation which leads to overexpression of the protein FGF23, a protein involved in the regulation of phosphate concentration in the blood. In XLH, FGF23 is produced in excess leading to depletion of phosphate in the blood, known as hypophosphataemia.¹

Individuals living with the disease may display a multitude of symptoms including short stature, limb deformities, bone and joint pain, oral abscesses, and hearing loss.¹ To manage this wide variety of symptoms, the disease is managed through multi-disciplinary teams.²

About CRYSVITA (burosumab)

CRYSVITA (burosumab) is a recombinant human monoclonal antibody (mAb) that binds to the protein fibroblast growth factor 23 (FGF23). This has the impact of inhibiting the action of FGF23, allowing phosphate regulation in the body to be restored.³

About Kyowa Kirin

Kyowa Kirin aims to discover novel medicines with life-changing value. As a Japan-based Global Specialty Pharmaceutical Company, we have invested in drug discovery and biotechnology innovation for more than 70 years and are currently working to engineer the next generation of antibodies and cell and gene therapies with the potential to help patients affected by severe and rare diseases. A shared commitment to our values, to sustainable growth, and to making people smile unites us across our four regions—Japan, Asia Pacific, North America, and EMEA/International.

You can learn more about the business of Kyowa Kirin at: <https://www.kyowakirin.com>

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References

¹Beck-Nielsen SS, et al. 2019. FGF23 and its role in X-linked hypophosphatemia-related morbidity. Orphanet Journal of Rare Diseases. 2022;14:1-25.

²Kubota T. X-linked hypophosphatemia transition and team management. Endocrines. 2022;3(3):411-418.

³European Medicines Agency. Summary of Product Characteristics (SmPC). CRYSVITA® (burosumab). 2023. Available [here](#). [Last accessed September 2024].