FOR MEDICAL MEDIA

News release

New Analysis Demonstrates Impaired Work Productivity and Increased Unemployment Rates in Adults with X-linked Hypophosphataemia (XLH)

- *Findings from XLH Disease Monitoring Program (DMP) published in Journal of Bone and Mineral Research*
- *The DMP is a global effort, supported by Kyowa Kirin, to advance scientific understanding and care of XLH through real-world research*
- *Additional findings to be shared at American Society for Bone and Mineral Research annual meeting in Toronto, Canada, 27-30 September 2024*

Galashiels and Marlow, United Kingdom., 10 September 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, announced today the publication of a new analysis showing low levels of full-time employment and impaired work productivity among working age adults enrolled in the X-linked hypophosphataemia (XLH) Disease Monitoring Program (DMP), a prospective, 10-year, observational study of adults and children with XLH in the US, Canada, and Latin America.[1]

XLH is a rare, genetic, progressive, phosphate-wasting disorder that can cause skeletal abnormalities, stiffness, pain, and impaired physical function.[2] This analysis of baseline data from the XLH DMP (n=281) showed that 31% of working age adults with XLH were not employed—a rate eight times higher than in the US general population and five times higher than in the EU— and 15% were receiving disability payments.[1,3,4]

Additionally, individuals with a higher number of past orthopaedic surgeries, and those with worse physical function, were less likely to be employed. Among those employed (n=193), the majority (60%) were working in light or sedentary roles and those in heavier work roles reported worse pain on average.[1]

“These findings highlight the substantial burden of XLH, which impacts multiple aspects of affected individuals' lives. Exploring real-world experiences helps to expand understanding of the disease beyond clinical endpoints to outcomes that are meaningful to patients,” said lead author, Professor Aliya Khan, MD, McMaster University, Canada. “Here, we learn that adults with XLH experienced challenges in the workplace. Understanding the factors underpinning this is an important consideration when planning patient care."

A disease monitoring programme provides an alternative to a traditional registry and extensive post-marketing studies and uses a collaborative, multi-stakeholder approach to monitor disease manifestations over an extended period, without limiting participants based on treatment received.

“Kyowa Kirin is committed to generating real-world evidence that builds greater understanding of the XLH patient experience and the effectiveness of treatment on outcomes relevant to patients and clinicians, in both the short- and long-term,” said Ben Johnson, study author and Director of Global Health Economics and Outcomes Research at Kyowa Kirin. “We look forward to sharing further findings from the XLH DMP and other real-world studies at the American Society for Bone and Mineral Research annual meeting in September.”

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About the XLH Disease Monitoring Program (DMP)[1]
The XLH DMP is an international, prospective, 10-year, longitudinal, observational study of adults and children with XLH (NCT03651505) designed to characterise XLH disease presentation and progression, and prospectively assess changes over time in biochemical, clinical, and patient/caregiver-reported outcomes in a representative population. The DMP steering committee includes clinical experts in XLH, patient advocates from the XLH Network, and representatives from Kyowa Kirin and Ultragenyx Pharmaceutical.

This study was designed to investigate the association of patient characteristics and work productivity; causality was not assessed and should not be inferred based on these findings. The study population was predominantly from the US (80.8%, with the remaining 19.2% from Brazil, Canada, and Chile), which may have different employment profiles from other countries, limiting the generalisability of the results outside the US.

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.[2]

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.[5] To manage this wide variety of symptoms, the disease is managed through multi-disciplinary teams.[6]

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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