News release

Kyowa Kirin Receives Positive CHMP Opinion for Use of CRYSVITA® ▼(burosumab) for the Treatment of Tumour-Induced Osteomalacia (TIO)

CRYSVITA could offer access to the first biologic treatment for EU patients with TIO who cannot undergo surgical removal of tumours

Tokyo, Japan, 27 June 2022 – Kyowa Kirin Co., Ltd. (TSE: 4151, Kyowa Kirin) today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has recommended that CRYSVITA® (burosumab) be approved for the treatment of FGF23-related hypophosphataemia in Tumour-Induced Osteomalacia (TIO) associated with phosphaturic mesenchymal tumours (PMTs) that cannot be curatively resected or localised in children and adolescents aged 1 to 17 years and in adults.[1] CRYSVITA is also already licensed for use in the rare disease X-Linked Hypophosphataemia (XLH), for children and adolescents between 1 and 17 years of age with radiographic evidence of bone disease, and in adults.[2]

Also known as oncogenic osteomalacia, TIO is an acquired disorder caused by typically small, slow-growing, benign PMTs.[3,4] It is a rare condition with less than 1000 cases reported in the medical literature,[4] which mainly affects adults and with a mean onset age of 40 – 45 years.[3,5] TIO is associated with progressive and debilitating musculoskeletal deficits,[6,7] ultimately having a detrimental impact on ability to perform daily activities, as well as on physical and social wellbeing.[8]

A cure for TIO can be achieved with complete surgical resection of the causative tumour(s), however, surgical resection is not always possible due to the location and difficulty in detecting tumours.[3,5] TIO may recur and persist following incomplete or unsuccessful surgical resection.[9]

If approved by the European Commission (EC), CRYSVITA will be the first biologic treatment available to European patients with TIO, which blocks the action of FGF23, restoring phosphate homeostasis.[2,10]

“Being diagnosed with a rare condition, like TIO, does present many challenges for patients and treating physicians, including the diagnostic process and the current lack of specific therapies. The unmet need for people living with TIO is clear, so this positive CHMP opinion is a very important step forward for those who cannot be cured by tumour removal and for the healthcare professionals supporting them,” said Professor Ralf Oheim, Department of Osteology and Biomechanics, University Medical Center Hamburg.

"For people diagnosed with TIO in Europe, we are a step closer to being able to deliver the first biologic treatment for those who cannot undergo surgical removal of tumours," said Abdul Mullick, President of Kyowa Kirin International. "This positive CHMP opinion is a much welcomed milestone for CRYSVITA, which is currently approved for use in Europe in adults and children with X-Linked Hypophosphataemia. I'm proud that Kyowa Kirin International is helping those living with TIO, as part of our extensive work in supporting those with rare diseases gain access to life changing therapies for their diseases."

Administered by a subcutaneous injection, CRYSVITA is a recombinant fully human monoclonal antibody that binds to and inhibits the activity of FGF23, restoring phosphate homeostasis.[2] The efficacy and safety of CRYSVITA have been demonstrated in two Phase II clinical trials published in the disease area of TIO.[11,12] CRYSVITA has been approved for clinical use in X-Linked Hypophosphataemia (XLH) across the European Union (EU) and Great Britain (GB) since 2018, and in this indication is presently approved for use in children and adolescents aged 1 and 17 years of age with radiographic evidence of bone disease, and in adults.[2]

The EC will review the CHMP recommendation and a final decision on the expansion of the recommended use for CRYSVITA for the treatment of FGF23-related hypophosphataemia in TIO associated with phosphaturic mesenchymal tumours (PMTs) that cannot be curatively resected or localised in children and adolescents aged 1 to 17 years and in adults is expected in the coming months. This means that use of CRYSVITA in this TIO indication is not currently approved in the EU or GB.

▼ This medicinal product is subject to additional monitoring.

About Tumour-Induced Osteomalacia (TIO)

TIO is characterised by chronic hypophosphataemia caused by tumour(s) secreting excess fibroblast growth factor 23 (FGF23),[3] which can lead to issues such as decreased intestinal phosphate absorption and compromised vitamin D activation.[3,4]

The most common signs and symptoms include bone pain, difficulty walking, pathological fractures, height loss and muscle weakness.[6] In TIO, muscle weakness and pain severely interfere with physical functioning, including standing up without assistance, walking and ability to work.[8] The pain in TIO also severely interferes with mood and moderately interferes with enjoyment of life for those living with it.[8]

TIO diagnosis is often missed and/or delayed and testing serum phosphate levels is important for diagnosis.[3] The only cure in TIO is complete removal of the causative tumour(s).[3] Pharmacological treatment should be considered in TIO cases where tumour(s) cannot be curatively resected or localised.[3] Restoring phosphate homeostasis is essential to improve the health of people living with TIO.[3]

About CRYSVITA® (burosumab) in TIO

CRYSVITA (burosumab) was created and developed by Kyowa Kirin and is a recombinant fully human monoclonal antibody that binds to and inhibits the activity of FGF23.[2] CRYSVITA blocks the action of FGF23, restoring phosphate homeostasis.[2]

The efficacy and safety of CRYSVITA have been demonstrated in two Phase 2 clinical trials published in the disease area of TIO.[11,12] CRYSVITA was well-tolerated and demonstrated an acceptable safety profile.[11,12]

CRYSVITA is presently indicated for the treatment of XLH in children and adolescents aged 1 to 17 years with radiographic evidence of bone disease, and in adults.[2] If approved by the European Commission, CRYSVITA would be indicated for the treatment of FGF23-related hypophosphataemia in TIO associated with PMTs that cannot be curatively resected or localised in children and adolescents aged 1 to 17 years and in adults.[1] CRYSVITA is given as a subcutaneous injection, every 4 weeks in adults and every 2 weeks in children and adolescents aged 1 to 17 years.[2]

CRYSVITA is currently approved for use in the treatment of TIO in a number of countries, including the United States[13] and Japan.[14]

Kyowa Kirin and Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE: Ultragenyx) have been collaborating in the development and commercialisation of CRYSVITA globally, based on the collaboration and licence agreement between Kyowa Kirin and Ultragenyx.

About Kyowa Kirin

Kyowa Kirin strives to create and deliver novel medicines with life-changing value. As a Japan based global specialty pharmaceutical company with a heritage of more than 70 years, the company applies cutting-edge science, including expertise in antibody research and engineering, to address the needs of patients across multiple therapeutic areas such as nephrology, oncology, immunology/allergy and neurology. Across its four regions – Japan, Asia Pacific, North America and EMEA/International – Kyowa Kirin focuses on its purpose, to make people smile, and is united by its shared values of commitment to life, teamwork, innovation and integrity.

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

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