

KYGEVVI® (doxecitine and doxribtimine) recommended for approval in the European Union as treatment for Thymidine Kinase 2 Deficiency (TK2d)

- **Positive CHMP Opinion:** If approved by the European Commission, KYGEVVI® (doxecitine and doxribtimine) would be the first and only treatment option in the European Union indicated for the treatment of paediatric and adult patients with genetically confirmed thymidine kinase 2 deficiency (TK2d) with an age of symptom onset on or before 12 years.¹
- **Burden of disease:** TK2d is an ultra-rare, life-threatening, genetic mitochondrial disease characterized by progressive and severe muscle weakness (myopathy).²

Brussels (Belgium) 30 January 2026 – UCB (Euronext Brussels: UCB), a global biopharmaceutical company, today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) adopted a positive opinion recommending granting marketing authorization under exceptional circumstances for KYGEVVI® (doxecitine and doxribtimine) for the treatment of pediatric and adult patients with genetically confirmed thymidine kinase 2 deficiency (TK2d) with an age of symptom onset on or before 12 years.¹ The final European Commission (EC) decision is anticipated in Q2 2026.

"This positive CHMP opinion marks a turning point in the treatment of TK2d, offering a new chapter of hope and a meaningful step forward for patients, families, and clinicians alike," said Donatello Crocetta, Chief Medical Officer at UCB. "We are deeply proud to be one step closer to being able to bring the first and only approved treatment for TK2d to the community."

"Doxecitine and doxribtimine treatment is a great example of how robust pre-clinical data can be translated into a clinical program in ultra-rare disease, impacting the natural history of a fatal disorder such as thymidine kinase 2 deficiency" said Caterina Garone, Associate Professor of Medical Genetics, University of Bologna, Italy. "The CHMP positive opinion is a significant step toward potential access to the first treatment option for TK2d in Europe."

Doxecitine and doxribtimine was supported through EMA's PRImity MEdicines (PRIME) scheme, which provides early and enhanced scientific and regulatory support to medicines that have a potential to address patients' unmet medical needs. Marketing authorization under exceptional circumstances may be granted to medicines where the applicant is unable to provide comprehensive data on efficacy and safety under normal conditions of use, because the condition to be treated is rare or because collection of full information is not possible or is unethical.

About TK2d

TK2d is an ultra-rare, life-threatening, genetic mitochondrial disease characterized by progressive (worsening over time) and severe muscle weakness (myopathy) with no approved treatment options in Europe beyond supportive care.^{2,3,4,5} TK2d can impact all parts of a person's daily life and wellbeing and can become worse over time and impact the ability to walk, eat and breathe independently, often necessitating around-the-clock caregiver support.^{3,4,6} It is often fatal, with those experiencing initial symptoms on or before the age of 12 years facing a high risk of premature death (often occurring within 3 years after symptom onset).⁷ It is estimated that the worldwide prevalence of TK2d is 1.64 [0.5, 3.1] cases per 1,000,000 people.⁸

Supportive data

Supportive data came from pooled data from two studies* of treatment with doxecitine and doxribtimine in patients with genetically confirmed TK2d with age of symptom onset \leq 12 years. These studies investigated the impact of treatment on functional outcomes (i.e. motor milestones, ventilatory support, feeding support) as well as survival.^{1,4,9,10} In the studies, the most common side effects associated with treatment were gastrointestinal disorders, including diarrhoea, vomiting, and abdominal pain.¹

About doxecitine and doxribtimine mechanism of action

Doxecitine and doxribtimine are incorporated into the mitochondrial DNA of skeletal muscle, restoring mitochondrial DNA copy number and improving skeletal muscle function in patients with TK2d.¹

**These two studies comprise 39 participants with an age of TK2d symptom onset \leq 12 years - the median age of TK2d symptom onset was 1.89 years (Q1, Q3 = 1.2, 2.7) and the median duration of treatment was 91.4 months (Q1, Q3 = 80.2, 117.8; all treated $>$ 5 years). Survival was compared to an external control group, after matching on Age of Symptom Onset Category. Information on functional outcomes in the treated patients (motor milestones, ventilatory support, and feeding support) was collected during the pre- and post-treatment period and analysed.*

For more information about the trials visit: <https://clinicaltrials.gov/study/NCT03845712> (TK0102/NCT03845712) and <https://clinicaltrials.gov/study/NCT03701568> (MT-1621-101/NCT03701568).

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About UCB

UCB, Brussels, Belgium (www.ucb.com) is a global biopharmaceutical company focused on the discovery and development of innovative medicines and solutions to transform the lives of people living with severe diseases of the immune system or of the central nervous system. With more than 9 000 people in approximately 40 countries, the company generated revenue of € 6.1 billion in 2024. UCB is listed on Euronext Brussels (symbol: UCB). Follow us on Twitter: @UCB_news

Forward-looking statements

This document contains forward-looking statements, including, without limitation, statements containing the words "potential", "believes", "anticipates", "expects", "intends", "plans", "seeks", "estimates", "may", "will",

“continue” and similar expressions. These forward-looking statements are based on current plans, estimates and beliefs of management. All statements, other than statements of historical facts, are statements that could be deemed forward-looking statements, including estimates of revenues, operating margins, capital expenditures, cash, other financial information, expected legal, arbitration, political, regulatory or clinical results or practices and other such estimates and results. By their nature, such forward-looking statements are not guaranteeing future performance and are subject to known and unknown risks, uncertainties, and assumptions which might cause the actual results, financial condition, performance or achievements of UCB, or industry results, to be materially different from any future results, performance, or achievements expressed or implied by such forward-looking statements contained in this document.

Important factors that could result in such differences include but are not limited to: global spread and impacts of wars, pandemics and terrorism, the general geopolitical environment, climate change, changes in general economic, business and competitive conditions, the inability to obtain necessary regulatory approvals or to obtain them on acceptable terms or within expected timing, costs associated with research and development, changes in the prospects for products in the pipeline or under development by UCB, effects of future judicial decisions or governmental investigations, safety, quality, data integrity or manufacturing issues, supply chain disruption and business continuity risks; potential or actual data security and data privacy breaches, or disruptions of our information technology systems, product liability claims, challenges to patent protection for products or product candidates, competition from other products including biosimilars or disruptive technologies/business models, changes in laws or regulations, exchange rate fluctuations, changes or uncertainties in tax laws or the administration of such laws, and hiring, retention and compliance of its employees. There is no guarantee that new product candidates will be discovered or identified in the pipeline, or that new indications for existing products will be developed and approved. Movement from concept to commercial product is uncertain; preclinical results do not guarantee safety and efficacy of product candidates in humans. So far, the complexity of the human body cannot be reproduced in computer models, cell culture systems or animal models. The length of the timing to complete clinical trials and to get regulatory approval for product marketing has varied in the past and UCB expects similar unpredictability going forward. Products or potential products which are the subject of partnerships, joint ventures or licensing collaborations may be subject to disputes between the partners or may prove to be not as safe, effective or commercially successful as UCB may have believed at the start of such partnership. UCB's efforts to acquire other products or companies and to integrate the operations of such acquired companies may not be as successful as UCB may have believed at the moment of acquisition. Also, UCB or others could discover safety, side effects or manufacturing problems with its products and/or devices after they are marketed. The discovery of significant problems with a product similar to one of UCB's products that implicate an entire class of products may have a material adverse effect on sales of the entire class of affected products. Moreover, sales may be impacted by international and domestic trends toward managed care and health care cost containment, including pricing pressure, political and public scrutiny, customer and prescriber patterns or practices, and the reimbursement policies imposed by third-party payers as well as legislation affecting biopharmaceutical pricing and reimbursement activities and outcomes. Finally, a breakdown, cyberattack or information security breach could compromise the confidentiality, integrity and availability of UCB's data and systems.

Given these uncertainties, the public is cautioned not to place any undue reliance on such forward-looking statements. These forward-looking statements are made only as of the date of this document, and do not reflect any potential impacts from the evolving event or risk as mentioned above as well as any other adversity, unless indicated otherwise. The company continues to follow the development diligently to assess the financial significance of these events, as the case may be, to UCB.

UCB expressly disclaims any obligation to update any forward-looking statements in this document, either to confirm the actual results or to report or reflect any change in its forward-looking statements with regard thereto or any change in events, conditions or circumstances on which any such statement is based, unless such statement is required pursuant to applicable laws and regulations.

References:

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