UCB News

UCB presents research at EUROMIT 2026 highlighting burden of care, need for earlier diagnosis, and impact of pyrimidine nucleoside therapy on people living with TK2d

- **Impact of disease progression underscores need for early diagnosis:** Survey of people living with TK2d found disease progression increased the need to make adaptations to assist with daily life and led to loss of independence and greater mental burden, highlighting the need for early diagnosis.[1]
- **Starting pyrimidine nucleoside therapy a positive turning point:** Survey participants reported the loss of mobility and/or muscle strength to be the most negative turning point in their disease progression.[2] Starting pyrimidine nucleoside therapy was the strongest positive turning point, enhancing impact on life of patients as the disease progresses.[2]
- **Burden of resource needs and costs in childhood-onset TK2d:** Data show that healthcare resource use and costs associated with the disease increase substantially as disease progresses.[3]

Brussels (Belgium) 2 June 2026, 0700: (CEST) – UCB (Euronext Brussels: UCB), a global biopharmaceutical company, is presenting its latest research on the ultra-rare disease, thymidine kinase 2 deficiency (TK2d) this week at the 2026 European Meeting on Mitochondrial Pathology (EUROMIT), Angers, France. The research highlights the increased burden, cost of care, and impact on the lives of patients as the disease progresses, underscoring the need for early diagnosis to aid appropriate support and treatment, as well as the perceived benefits of pyrimidine nucleoside therapy.[1,2,3]

UCB aims to help close gaps in awareness and understanding and help provide solutions that address TK2d's impact on patients and families, and these new data on disease progression, lived experience and burden of care add significantly to this area. Donatello Crocetta, Chief Medical Officer, UCB, explains: "Listening to people living with an ultra-rare condition like TK2d is essential to understanding its true impact, including how that burden can increase over time and how timely diagnosis and treatment help to ease that burden. At UCB, putting the experiences of people living with TK2d at the heart of our work helps our efforts to address the unmet needs that matter most to them and their families."

Key data highlights

Patient-reported data illustrate early TK2d symptoms, the impact of disease progression on the lives of patients and the importance of early diagnosis[1]

An online forum involving 10 patients with genetically confirmed TK2d, or proxy caregivers, explored early symptoms, disease progression, and the importance of early diagnosis. Overall, worsening TK2d symptoms were reported to affect mobility, daily activities, and led to decreased independence.

- Most participants (n=8/10) reported first symptoms at ≤12 years, primarily lower and upper muscle weakness, including falls and "floppiness."
- As symptoms progressed, including the emergence of respiratory and swallowing difficulties, participants described greater need for daily-life adaptations, increased mental burden, and greater loss of independence and social, educational and professional opportunities.
- Fatigue and low energy levels (n=7/9), respiratory and swallowing difficulties (n=5/9), and worsening muscular/neurological symptoms (n=4/9) were reported as factors leading to a "challenging day."

Patient insights highlight turning points across the TK2d disease journey such as the burden of functional decline and the benefits of treatment [2]

The online forum participants also described pivotal moments in their disease journey deepening the understanding of the lived experience of TK2d.

- All participants identified loss of mobility and/or muscle strength as the most significant negative turning point in disease progression. Respiratory difficulties (n=7/10) and swallowing difficulties (n=5/10) were also identified as negative turning points.
- Among patients receiving pyrimidine nucleoside therapy via compassionate use, most (n=7/8) reported treatment initiation as the strongest positive turning point, with perceived improvements in muscle strength, motor function, respiratory function and swallowing capacity - helping patients regain some independence and improving their quality of life.
- Other positive turning points included supportive mobility aids/equipment (n=4/10) and receiving a TK2d diagnosis (n=4/10).

New analysis underscores the healthcare resource use and costs associated with supporting children with TK2d[3]

A targeted literature review and expert input from five European specialists highlighted the substantial burden of early-onset TK2d and the increase in costs and healthcare resource use (HCRU) as the disease progresses, displaying the profound impact of TK2d on children and their caregivers.

- Children with TK2d were reported to have a life expectancy of approximately 4 years if symptoms start at age ≤12 years and they experience exponential functional decline requiring ventilation, tube feeding, and intensive multidisciplinary care.
- Children with TK2d require at least one annual visit to four or more healthcare professionals, with care needs increasing from home-based adaptations to round-the-clock care and eventual intensive care unit placement.
- Families were also reported to face workforce loss and high out-of-pocket costs for travel, home modifications and assistive devices.
- TK2d in children was considered more severe than spinal muscular atrophy (SMA) types 2 & 3 and Duchenne muscular dystrophy, with costs expected to surpass the cost of care for SMA types 2 & 3.

UCB is also supporting a medical education symposium at the congress, titled "Changing the natural history of TK2d: Navigating the pathway to earlier diagnosis and improved outcomes", 2 June, 12:40 – 13:30 at the Amphi Jardin level 2. The symposium will discuss the natural history of TK2d, best practices for earlier diagnosis of suspected TK2d cases, and practical considerations for optimized management.

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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 9000 people in approximately 40 countries, the company generated revenue of €7.7 billion in 2025. UCB is listed on Euronext Brussels (symbol: UCB).

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.

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KYGEVVI® ▼ (doxecitine and doxribtimine) EU/EEA** Important Safety Information[4]

KYGEVVI is 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.

Increase in Liver Transaminases
Elevated liver enzymes and liver dysfunction/failure have been observed as a clinical manifestation of TK2d. In clinical studies elevations in alanine aminotransferase [ALT] and/or aspartate aminotransferase [AST] have occurred in patients with TK2d following treatment with KYGEVVI. Transaminase levels should be checked prior to initiation of treatment, and changes in liver function monitored periodically during treatment with KYGEVVI and according to routine patient management.

Gastrointestinal disturbances
Gastrointestinal disturbances such as diarrhoea, vomiting, and abdominal pain (including abdominal pain upper) are very commonly reported adverse reactions with doxecitine and doxribtimine treatment. In the pooled safety population 37 out of 50 participants (74%) experienced diarrhoea early after treatment initiation (<3 months). The majority of events of diarrhoea were mild to moderate in severity, and were generally self-limiting or improved with temporary dose reduction. Of 133 events of diarrhoea, 12% (16/133) required dose reduction with a median duration of 80 days (Q1, Q3=33.0, 201.5). None of the 50 participants discontinued due to gastrointestinal disturbances, including diarrhoea.

Please consult the summary of product characteristics in relation to other side effects, full safety and prescribing information.

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***EU/EEA means European Union/European Economic Area*

▼This medicinal product is subject to additional monitoring. This will allow quick identification of new safety information. Healthcare professionals are asked to report any suspected adverse reactions.

References:

1. Waller K, et al. Early symptoms and disease progression in thymidine kinase 2 deficiency: patient perspectives from an online forum. Poster presented at EUROMIT 2026, May 30 – June 4, Angers, France. Poster D3-P088.
2. Karaa A, et al. The thymidine kinase 2 deficiency trajectory: patient-reported turning points. Poster presented at EUROMIT 2026, May 30 – June 4, Angers, France. Poster D3-P087.
3. Roberts M, et al. Healthcare resource use and costs required to support children with thymidine kinase 2 deficiency. Poster presented at EUROMIT 2026, May 30 – June 4, Angers, France. Poster D4-P027.
4. KYGEVVI® EU SmPC. https://www.ema.europa.eu/en/documents/product-information/kygevvi-epar-product-information_en.pdf. Accessed May 2026.

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