29 January 2026
EMA/CHMP/384297/2025
Committee for Medicinal Products for Human Use (CHMP)

**Summary of opinion[1] (initial authorisation)**

# Kygevvi

## doxecitine / doxribtimine

On 29 January 2026, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation under exceptional circumstances[2] for the medicinal product Kygevvi[3], intended for the treatment of adults and children with genetically confirmed thymidine kinase 2 deficiency (TK2d) with an age of symptom onset at or before 12 years.

The applicant for this medicinal product is UCB Pharma S.A.

Kygevvi is a PRIME medicine. This scheme optimises the development and accelerates the evaluation of medicines that fulfil an unmet medical need, helping them to reach patients sooner.

Kygevvi will be available as a 2 g / 2 g powder for oral solution. The active substances of Kygevvi, doxecitine and doxribtimine, are pyrimidine nucleosides (ATC code: A16AX29). 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.

The benefit of Kygevvi is a reduction in the number of motor milestones lost or even a regain of motor milestones, as observed in a pooled analysis of data from a retrospective chart review study (MT-1621-101) and an open-label, single-arm clinical study (TK0102) in patients with TK2d treated with pyrimidine nucleos(t)ides. The most common side effects with Kygevvi are gastrointestinal disorders, including diarrhoea, vomiting, and abdominal pain.

The full indication is:

> 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.

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] In exceptional circumstances, an authorisation may be granted subject to certain specific obligations, to be reviewed annually. This happens when the applicant can show that they are unable to provide comprehensive data on the efficacy and safety of the medicinal product, due to the rarity of the condition it is intended for, limited scientific knowledge in the area concerned, or ethical considerations involved in the collection of such data.
[3] This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained

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Kygevvi is intended for use with the instructions and supervision of specialist healthcare professionals experienced in the management of patients with mitochondrial disorders.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published on the EMA website in all official European Union languages after the marketing authorisation has been granted by the European Commission.