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Kygevvi (doxecitine / doxribtimine)

An overview of Kygevvi and why it is authorised in the EU

What is Kygevvi and what is it used for?

Kygevvi is a medicine used to treat people with thymidine kinase 2 deficiency (TK2d), confirmed by a genetic test, whose symptoms of the disease started at or before 12 years of age. TK2d is a condition caused by mutations (changes) in the *TK2* gene which lead to progressive muscle weakness, loss of ability to move and walk, breathing difficulties and a shortened life expectancy.

TK2d is rare, and Kygevvi was designated an 'orphan medicine' (a medicine used in rare diseases) on 20 April 2017. Further information on the orphan designation can be found on the EMA website.

Kygevvi contains the active substances doxecitine and doxribtimine.

How is Kygevvi used?

Kygevvi can only be obtained with a prescription, and treatment should be supervised by a doctor experienced in conditions affecting mitochondria, the energy-producing components within cells.

Kygevvi is available as a powder to be mixed in water and taken by mouth three times a day.

For more information about using Kygevvi, see the package leaflet or contact your doctor or pharmacist.

How does Kygevvi work?

The TK2 enzyme helps produce and maintain the DNA inside mitochondria. In people with TK2d, mutations in TK2 prevent it from working properly. As a result, mitochondria do not work as they should, and muscles cannot produce enough energy, leading to progressive muscle weakness.

The way Kygevvi works in people has not been confirmed. However, studies in animals suggest that its active substances, doxecitine and doxribtimine (DNA building blocks), are taken up by muscle cells and become part of mitochondrial DNA. This helps improve the production and maintenance of mitochondrial DNA. In this way, Kygevvi is expected to compensate for reduced TK2 activity and help slow the worsening of muscle weakness in people with the condition.

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What benefits of Kygevvi have been shown in studies?

In adults and children with TK2d, confirmed by genetic testing, whose symptoms of the disease started at or before 12 years of age, treatment with Kygevvi allowed people to regain motor milestones.

Because TK2d is a rare disease, there are too few patients to carry out a study comparing Kygevvi with another treatment or with placebo (a dummy treatment). The effects of Kygevvi were therefore assessed by looking at patients' medical records and at the results of a study in which all patients received Kygevvi. In total, 39 patients whose symptoms started at or before 12 years of age were included in the evaluation.

The effect of treatment was assessed by comparing motor milestones, such as the ability to sit, stand or walk, before and after starting Kygevvi. During treatment, around 84% of patients (26 out of 31) regained at least one motor milestone, while around 26% (10 out of 38) lost at least one motor milestone.

What are the side effects and restrictions with Kygevvi?

For the full list of side effects and restrictions with Kygevvi, see the package leaflet.

The most common side effects with Kygevvi (which may affect more than 1 in 10 people) include diarrhoea, vomiting and abdominal (belly) pain.

Why is Kygevvi authorised in the EU?

At the time of authorisation, there was no treatment authorised for TK2d, and care was limited to supportive measures. These included providing nutrition through a feeding tube, physiotherapy to support movement and breathing support using a ventilator.

Kygevvi was shown to allow patients whose symptoms of the disease started at or before 12 years of age to regain motor milestones. Although there was some uncertainty about the size of the effect, regaining motor milestones is rare in the natural course of the disease. It is therefore likely that regain in motor milestones was due to treatment with Kygevvi. The available data could not show whether Kygevvi has an effect on how long patients live. Further studies will provide more information to address these uncertainties.

The most common side effects were related to the gut and were considered manageable.

The European Medicines Agency therefore decided that Kygevvi's benefits are greater than its risks and it can be authorised for use in the EU.

Kygevvi has been authorised under exceptional circumstances. This is because it has not been possible to obtain complete information about Kygevvi due to the rarity of the disease. The company must provide further data on Kygevvi. It must submit the results of a new study to confirm the safety and effectiveness of the medicine. Every year, EMA will review any new information that becomes available.

What measures are being taken to ensure the safe and effective use of Kygevvi?

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Kygevvi have been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Kygevvi are continuously monitored. Suspected side effects reported with Kygevvi are carefully evaluated and any necessary action taken to protect patients.

Other information about Kygevvi

Kygevvi received a marketing authorisation under exceptional circumstances valid throughout the EU on 26 March 2026.

Further information on Kygevvi can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/kygevvi.

This overview was last updated in 03-2026.