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Withdrawal of application to change the marketing authorisation for Kaftrio (ivacaftor/tezacaftor/elexacaftor)

Vertex Pharmaceuticals (Ireland) Limited withdrew its application for the use of Kaftrio granules in combination with ivacaftor in children aged 1 year and older with cystic fibrosis.

The company withdrew the application on 1 September 2026.

What is Kaftrio and what is it used for?

Kaftrio is a medicine used to treat patients aged 2 years and above who have cystic fibrosis, an inherited disease that has severe effects on the lungs, the digestive system and other organs.

Cystic fibrosis can be caused by various mutations (changes) in the gene for a protein called cystic fibrosis transmembrane conductance regulator (CFTR). People have two copies of this gene, one inherited from each parent and the disease only occurs when there is a mutation in both copies.

Kaftrio is used in combination with ivacaftor in patients with cystic fibrosis who have at least one non-class I mutation in the *CFTR* gene.

Kaftrio is available as tablets and granules to be taken by mouth.

The medicine contains the active substances ivacaftor, tezacaftor and elexacaftor. It has been authorised in the EU since August 2020.

Kaftrio was designated an 'orphan medicine' (a medicine used in rare diseases) on 14 December 2018. Further information on the orphan designation can be found here: https://www.ema.europa.eu/en/medicines/human/orphan-designations/eu3182117

Further information on Kaftrio's current uses can be found on the Agency's website: ema.europa.eu/en/medicines/human/EPAR/Kaftrio.

What change had the company applied for?

The company applied to extend the use of Kaftrio in combination with ivacaftor to children aged 1 year and older with cystic fibrosis who have at least one non-class I mutation in the *CFTR* gene, together with a new dosage strength for the granule formulation.

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How does Kaftrio work?

Cystic fibrosis is caused by mutations in the *CFTR* gene. This gene leads to the production of the CFTR protein, which works on the surface of cells to regulate the production of mucus in the lungs and digestive juices in the gut. The mutations reduce the number of CFTR proteins on the cell surface or affect the way the protein works, resulting in mucus and digestive fluids being too thick. This leads to blockages, inflammation, increased risk of lung infections, and poor digestion and growth.

Two of the active substances in Kaftrio, elexacaftor and tezacaftor, increase the number of CFTR proteins on the cell surface, while the other, ivacaftor, improves the activity of the defective CFTR protein. These actions combine to make lung mucus and digestive juices less thick, thereby helping to relieve symptoms of the disease.

What did the company present to support its application?

Available data on the effectiveness and safety of ivacaftor/tezacaftor/elexacaftor in combination with ivacaftor in older children and adults were presented to support the medicine use in younger children. The company also provided clinical data from a study in children aged between 1 and 2 years with cystic fibrosis, which looked at how the medicine is absorbed, modified and removed from the body, as well as its effects and safety.

How far into the evaluation was the application when it was withdrawn?

The application was withdrawn after the European Medicines Agency had evaluated the initial information from the company and had prepared questions for the company. The company had not responded to the questions at the time of the withdrawal.

What did the Agency recommend at that time?

As the Agency was still evaluating the initial information from the company, it had not yet made any recommendations.

What were the reasons given by the company for withdrawing the application?

In its letter notifying the Agency of the withdrawal, the company stated that it withdrew its application due to the Agency's preliminary feedback that additional information would be required to support this extension of indication.

Does this withdrawal affect patients in clinical trials?

The company informed the Agency that there are no consequences for patients in clinical trials using Kaftrio.

If your child is in a clinical trial and you need more information about their treatment, speak with their clinical trial doctor.

What is happening with Kaftrio in its authorised uses?

There are no consequences on the use of Kaftrio in its authorised uses.