National Centre for Pharmacoeconomics
NCPE Ireland

NCPE Plain English Summary

Drug name: Kaftrio *(pronounced: Kaf - trio)* is a combination of ivacaftor + tezacaftor + elexacaftor for the treatment of cystic fibrosis patients aged 6 years and older who are heterozygous for the F508del mutation and either a minimal function mutation or an unknown mutation in the cystic fibrosis transmembrane conductance regulator gene.

Brand name: Kaftrio®

What is the NCPE?

The National Centre for Pharmacoeconomics (NCPE) is a team of experts who look at the health benefits and costs of medicines. The HSE asks us to advise on whether or not a new medicine is good value for money. We give unbiased advice to help the HSE provide the most effective, safe and cost-effective (value for money) treatments for patients.

How do we make our recommendations?

Our main focus is on the health benefits and cost effectiveness of a medicine. We look at the wider costs and health benefits associated with a new medicine, for example:

- Does the new medicine work better than other treatments available in Ireland?
- Is the new medicine easier to give or easier to take compared with other treatments available in Ireland?
- Does the new medicine reduce the need for patients to be hospitalised?
- Does the new medicine improve the quality of a patient's life over other treatments available in Ireland?
- Will the new medicine save resources elsewhere within the health system?

We review the information from clinical trials along with the cost and value for money data presented by the pharmaceutical company. We ask doctors and other healthcare professionals for advice about any health benefits of the new medicine compared with current treatments. We also ask patient organisations to send us their views on how the new drug may improve patients' day-to-day experience of living with a disease.

What is Kaftrio used for?

Kaftrio is administered as two tablets daily (in the morning time) in addition to one tablet of ivacaftor daily (in the evening time) for the treatment of cystic fibrosis patients with a specific

cystic fibrosis gene mutation known as the F508del mutation. The beneficial effects of Kaftrio plus ivacaftor in children aged 6 years and older include improvements in lung function, weight gain and quality of life. Patients will continue to receive symptom-based therapies as required.

What recommendation has the NCPE made to the HSE?

Kaftrio plus ivacaftor daily treatment is expensive and so we have recommended that the HSE should consider funding Kaftrio for the treatment of cystic fibrosis patients aged 6 years and older if the cost-effectiveness (value for money) can be improved when compared with current treatments. The HSE will consider our recommendation and make the final decision about reimbursement (funding). When making the funding decision, the HSE will also consider the additional criteria outlined in the Health (Pricing and Supply of Medical Goods) Act 2013.

Why did we make this recommendation?

After reviewing the data presented by the pharmaceutical company, we recommend that the HSE considers funding Kaftrio plus ivacaftor provided that it obtains the drug therapy at a price which improves the value for money associated with this treatment.

Next steps

When the HSE receives our recommendation, it will look at all the relevant data about Keftrio. The HSE makes the final decision on reimbursement.

Where can I get more information?

You can get more information about Kaftrio from the following online options:

- the NCPE Technical Summary Document
- Searching for Kaftrio on our website (www.ncpe.ie);
- searching for Kaftrio on the European Medicines Agency (EMA) website (www.ema.europa.eu).

Please refer to the NCPE website for updated information on the reimbursement status of this medicine.

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