25 April 2025
EMA/CHMP/131190/2025
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Alyftrek

deutivacaftor / tezacaftor / vanzacaftor

On 25 April 2025, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Alyftrek[2], intended for the treatment of cystic fibrosis in people aged 6 years and older who have at least one non-class I mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene.

The applicant for this medicinal product is Vertex Pharmaceuticals (Ireland) Limited.

Alyftrek will be available as 50 mg / 20 mg / 4 mg and 125 mg / 50 mg / 10 mg film-coated tablets. The active substances of Alyftrek are deutivacaftor / tezacaftor / vanzacaftor (ATC code: R07AX33). Vanzacaftor and tezacaftor, are CFTR correctors that bind to different sites on the CFTR protein, leading to an increase in the amount of CFTR protein on the cell surface; deutivacaftor improves the activity of the defective CFTR protein at the cell surface. These combined actions make lung mucus and digestive juices less thick, thereby helping to relieve symptoms of the disease.

The benefits of Alyftrek are improved lung function, as measured by percent predicted FEV1 (forced expiratory volume in one second), after 24 weeks of treatment. Two phase 3, randomised, double-blind clinical studies showed Alyftrek to be as effective as Kaftrio in patients with cystic fibrosis and non-class I mutations aged 12 years and older.

A supportive, single arm, open-label study in children aged 6-11 years was also submitted. Most children harboured an F508del mutation.

The most common side effects are headache and diarrhoea.

The full indication is:

Alyftrek tablets are indicated for the treatment of cystic fibrosis (CF) in people aged 6 years and older who have at least one non-class I mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.

[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] 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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Alyftrek should be prescribed by physicians experienced in the treatment of cystic fibrosis.

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.