EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Press Office

Press release

European Medicines Agency recommends first-in-class medicine for treatment of cystic fibrosis

New medicine offers therapeutic innovation for treatment of cystic fibrosis; review under accelerated assessment

The European Medicines Agency has recommended Kalydeco (ivacaftor), an orphan-designated medicine, for the treatment of cystic fibrosis in patients age 6 years and older who have a *G551D* mutation in the cystic fibrosis transmembrane regulator (*CFTR)* gene.

The Agency's Committee for Medicinal Products for Human Use (CHMP) reviewed Kalydeco under accelerated assessment, in 150 days. Accelerated assessment is one of the Agency's tools to speed up access by patients to new medicines that are of major public health interest.

Kalydeco offers an innovative therapeutic approach for patients with cystic fibrosis: it is the first treatment that targets the underlying mechanism of the disease, by restoring the function of the mutated CFTR protein. Currently available therapies for patients with cystic fibrosis only address the consequences of the disease, not the underlying defect. Clinical studies showed that Kalydeco improved pulmonary function in cystic fibrosis patients with the specific *G551D-CFTR* mutation.

Cystic fibrosis is a rare, life-threatening genetic disorder affecting around 60,000 people in the European Union. It is caused by a mutation of the *CFTR* gene, which regulates salt and water transport in the body. The *CFTR* mutation allows too much salt and water into cells. This results in a build-up of thick, sticky mucus in the body's tubes and passageways. These blockages damage the lungs, digestive system and other organs. Symptoms start in early childhood and include persistent cough, recurring chest and lung infections and poor weight gain.

The most common side effects observed with Kalydeco are abdominal pain, diarrhoea, dizziness, rash, upper respiratory tract reactions (including upper respiratory tract infection, nasal congestion, pharyngeal erythema, oropharyngeal pain, rhinitis, sinus congestion and nasopharyngitis), headache and bacteria in sputum. There are no long-term safety data for this product and, as for all newly approved medicines, it will be kept under close review.

The Agency has so far recommended approval for eight medicines under accelerated assessment, three of which are for orphan-designated medicines.

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The Agency's recommendation is being sent to the European Commission for the adoption of a decision.

Notes

1. This press release, together with all related documents, is available on the Agency's website at: LINK
2. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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