24 May 2012
EMA/CHMP/311973/2012
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Kalydeco

ivacaftor

On 24 May 2012, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Kalydeco, 150 mg, film-coated tablet intended for the treatment of cystic fibrosis (CF) in patients age 6 years and older who have a G551D mutation in the CFTR (cystic fibrosis transmembrane conductance regulator) gene. Kalydeco was designated as an orphan medicinal product on 08 July 2008. The applicant for this medicinal product is Vertex Pharmaceuticals (U.K.) Ltd. They may request a re-examination of any CHMP opinion, provided they notify the European Medicines Agency in writing of their intention within 15 days of receipt of the opinion.

The active substance of Kalydeco is ivacaftor, a selective CFTR modulator, which restores the function of a defective CFTR protein by increasing CFTR channel gating to enhance chloride transport.

The benefits with Kalydeco are its ability to improve pulmonary function (measured as the absolute change from baseline in percent predicted FEV_1) in patients with cystic fibrosis and G551D mutation in at least one allele of the CFTR gene. Other beneficial effects demonstrated in the clinical studies were decrease rate of pulmonary exacerbations and increase in body weight. The most common side effects 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.

A pharmacovigilance plan for Kalydeco will be implemented as part of the marketing authorisation.

The approved indication is: the treatment of cystic fibrosis (CF) in patients age 6 years and older who have a G551D mutation in the CFTR gene. It is proposed that Kalydeco 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 in the European public assessment report (EPAR) and

[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.

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made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

The CHMP, on the basis of quality, safety and efficacy data submitted, considers there to be a favourable benefit-to-risk balance for Kalydeco and therefore recommends the granting of the marketing authorisation.