24 September 2015
EMA/CHMP/619312/2015
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (post authorisation)

Kalydeco

ivacaftor

On 24 September 2015, the Committee for Medicinal Products for Human Use (CHMP) adopted two positive opinions recommending changes to the terms of the marketing authorisation for the medicinal product Kalydeco. The marketing authorisation holder for this medicinal product is Vertex Pharmaceuticals (Europe) Ltd.

For the currently available 150 mg film-coated tablets, the CHMP recommended extending the indication to include:

"treatment of patients with cystic fibrosis (CF) aged 18 years and older who have an *R117H* mutation in the *CFTR* gene (see sections 4.4 and 5.1)."

In addition, the CHMP recommended extending the marketing authorisation for Kalydeco to include two new presentations: 50 mg and 75 mg granules in sachet. The granules are for use in children aged 2 years and older in the previously authorised indication.

For information, the full indication for Kalydeco tablets will be:

"treatment of patients with cystic fibrosis (CF) aged 6 years and older and weighing 25 kg or more who have one of the following gating (class III) mutations in the *CFTR* gene: *G551D*, *G1244E*, *G1349D*, *G178R*, *G551S*, *S1251N*, *S1255P*, *S549N* or *S549R* (see sections 4.4 and 5.1).

"treatment of patients with cystic fibrosis (CF) aged 18 years and older who have an *R117H* mutation in the *CFTR* gene (see sections 4.4 and 5.1)."

The full indication for Kalydeco granules will be:

"treatment of children with cystic fibrosis (CF) aged *2 years* and older and weighing less than 25 kg who have one of the following gating (class III) mutations in the *CFTR* gene: *G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N* or *S549R* (see sections 4.4 and 5.1)."

Detailed recommendations for the use of this product will be described in the updated summary of product characteristics (SmPC), which will be published in the revised European public assessment report (EPAR),

[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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and will be available in all official European Union languages after a decision on this change to the marketing authorisation has been granted by the European Commission.