27 February 2025
EMA/CHMP/64893/2025
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (post authorisation)

Kalydeco

ivacaftor

On 27 February 2025, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending a change to the terms of the marketing authorisation for the medicinal product Kalydeco. The marketing authorisation holder for this medicinal product is Vertex Pharmaceuticals (Ireland) Limited.

The CHMP adopted extensions to the existing indications for Kalydeco film-coated tablets and granules in sachets to include treatment of patients with at least one non-class I *CFTR* mutation, as follows:[2]

Kalydeco tablets are indicated:

- In a combination regimen with ivacaftor/tezacaftor/elexacaftor tablets for the treatment of adults, adolescents and children aged 6 years and older with cystic fibrosis (CF) who have at least one **non-class I** ~~*F508del*~~ mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene (see section**s 4.2 and** 5.1).

Kalydeco granules are indicated:

- In a combination regimen with ivacaftor/tezacaftor/elexacaftor for the treatment of cystic fibrosis (CF) in paediatric patients aged 2 to less than 6 years who have at least one **non-class I** ~~*F508del*~~ mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene (see section**s 4.2 and** 5.1).

For information, the full indications for Kalydeco will be as follows:

Kalydeco tablets are indicated:

- As monotherapy for the treatment of adults, adolescents, and children aged 6 years and older and weighing 25 kg or more with cystic fibrosis (CF) who have an *R117H CFTR* mutation or one of the following gating (class III) mutations in the cystic fibrosis

[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] New text in bold, removed text as strikethrough

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transmembrane conductance regulator (*CFTR*) gene: *G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N or S549R* (see sections 4.4 and 5.1).

- In a combination regimen with tezacaftor/ivacaftor tablets for the treatment of adults, adolescents, and children aged 6 years and older with cystic fibrosis (CF) who are homozygous for the *F508del* mutation or who are heterozygous for the *F508del* mutation and have one of the following mutations in the *CFTR* gene: *P67L, R117C, L206W, R352Q, A455E, D579G, 711+3A→G, S945L, S977F, R1070W, D1152H, 2789+5G→A, 3272-26A→G, and 3849+10kbC→T*.
- In a combination regimen with ivacaftor/tezacaftor/elexacaftor tablets for the treatment adults, adolescents and children aged 6 years and older who have at least one non-class I mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene.

Kalydeco granules are indicated:

- As monotherapy for the treatment of infants aged at least 1 month, toddlers and children weighing 3 kg to less than 25 kg with cystic fibrosis (CF) who have an *R117H CFTR* mutation or one of the following gating (class III) mutations in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene: *G551D*, *G1244E*, *G1349D*, *G178R*, *G551S*, *S1251N*, *S1255P*, *S549N* or *S549R* (see sections 4.4 and 5.1).
- In a combination regimen with ivacaftor/tezacaftor/elexacaftor for the treatment of cystic fibrosis (CF) in paediatric patients aged 2 to less than 6 years who have at least one non-class I mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene (see sections 4.2 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 on the EMA website in all official European Union languages after a decision on this change to the marketing authorisation has been granted by the European Commission.