17 September 2020
EMA/CHMP/481882/2020
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (post authorisation)

Kalydeco

ivacaftor

On 17 September 2020, 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 recommended the approval of a new 75-mg strength for Kalydeco tablets and an extension to the existing indication in combination with Symkevi (tezacaftor/ivacaftor) to allow use in children from 6 years of age.

For information, the full indication for Kalydeco tablets will be as follows:[2]

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 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 ~~100 mg~~/ivacaftor ~~150 mg~~ tablets for the treatment of adults**,** ~~and~~ adolescents**, and children** aged **6** ~~12~~ 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 75 mg/tezacaftor 50 mg/elexacaftor 100 mg tablets for the treatment of adults and adolescents aged 12 years and older with cystic fibrosis (CF) who are homozygous for the *F508del* mutation in the *CFTR* gene or heterozygous for *F508del* in the *CFTR*

[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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gene with a minimal function (MF) mutation (see section 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), 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.