



30 April 2020
EMA/207923/2020
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion¹ (post authorisation)

Kalydeco ivacaftor

On 30 April 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 adopted a change to the indication for Kalydeco tablets as follows:²

Kalydeco tablets are indicated 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 CFTR gene: *G551D*, *G1244E*, *G1349D*, *G178R*, *G551S*, *S1251N*, *S1255P*, *S549N* or *S549R* (see sections 4.4 and 5.1).

~~Kalydeco tablets are also indicated for the treatment of adults, adolescents, and children aged 18 years and older and weighing 25 kg or more with cystic fibrosis (CF) who have an R117H mutation in the CFTR gene (see sections 4.4 and 5.1).~~

Kalydeco tablets are also indicated in a combination regimen with tezacaftor 100 mg/ivacaftor 150 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 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*.

The CHMP also adopted a change to the indication for Kalydeco granules as follows:

Kalydeco granules are indicated for the treatment of infants aged at least 6 months, toddlers and children weighing 5 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 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

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



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.