



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

25 March 2021
EMA/177576/2021
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion¹ (post authorisation)

Kalydeco ivacaftor

On 25 March 2021, 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 an extension to the existing indication as follows:²

Kalydeco tablets are indicated in a combination regimen with ivacaftor /tezacaftor /elexacaftor tablets for the treatment of adults and adolescents aged 12 years and older with cystic fibrosis (CF) **who have at least one** ~~are homozygous for the F508del mutation in the CFTR gene or heterozygous for F508del and have a minimal function (MF) mutation in the CFTR gene~~ (see section 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 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 of adults and adolescents aged 12 years and older with cystic fibrosis (CF) who have at least one *F508del*

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 ~~striketrough~~



mutation in the *CFTR* gene (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.