22 February 2024
EMA/CHMP/65752/2024
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (post authorisation)

Kalydeco

ivacaftor

On 22 February 2024, 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 presentation of Kalydeco granules (13.4 mg granules in sachet) and an extension to the existing indication to allow use in children from 1 month of age.

For information, the full indications for Kalydeco granules will be as follows[2]:

> Kalydeco granules are indicated:
>
> As monotherapy for the treatment of infants aged at least **1 month** ~~4 months~~, toddlers and children weighing ~~5~~ **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 *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 *F508del* mutation in the *CFTR* gene (see section 5.1).

For information, the indications for Kalydeco tablets remain unchanged and are provided in the summary of product characteristics (SmPC).

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.

[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 in strikethrough

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