17 October 2019
EMA/567043/2019
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (post authorisation)

Kalydeco

ivacaftor

On 17 October 2019, 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 approval of a new 25-mg strength, for Kalydeco granules, and an extension to the existing indication to allow use in infants from 6 months of age and weighing from 5 kg to less than 25 kg.

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

"Kalydeco granules are indicated for the treatment of ~~children with cystic fibrosis (CF)~~**infants** aged ~~12~~ **at least 6** months**, toddlers** and ~~older and~~ **children** weighing ~~7~~**5** kg to less than 25 kg **with cystic fibrosis (CF)** who have 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 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, deleted text in strikethrough

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

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