

Final Appraisal Recommendation

Advice No: 3716 – December 2016

Ivacaftor (Kalydeco[®]) 50 mg and 75 mg granules

Limited submission by Vertex Pharmaceuticals (Europe) Ltd

Recommendation of AWMSG

Ivacaftor (Kalydeco[®]) granules are recommended as an option for use within NHS Wales for the treatment of children with cystic fibrosis (CF) aged 2 years to less than 6 years and weighing less than 25 kg who have one of the following gating (class III) mutations in the CF transmembrane conductance regulator (CFTR) gene: G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N or S549R.

This recommendation applies only in circumstances where the approved Wales Patient Access Scheme (WPAS) is utilised or where the list/contract price is equivalent or lower than the WPAS price.

Additional note(s):

- Please refer to the Summary of Product Characteristics for the full licensed indication.
- AWMSG considered that ivacaftor (Kalydeco[®]) satisfied the AWMSG criteria for ultra-orphan status.

In reaching the above recommendation AWMSG has taken account of the appraisal documentation prepared by the AWMSG Secretariat (reference number 2770), which includes the AWMSG Secretariat Assessment Report (ASAR), the Preliminary Appraisal Recommendation (PAR) and the applicant company's response to the PAR, clinical expert opinion (where available), the views of patients/patient carers (where available) and the lay member perspective.

This recommendation has been ratified by Welsh Government and will be considered for review every three years.

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All Wales Medicines Strategy Group Final Appraisal Recommendation – 3716:
Ivacaftor (Kalydeco[®]) 50 mg and 75 mg granules. December 2016.



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