All Wales Medicines Strategy Group

Grŵp Strategaeth Meddyginiaethau Cymru Gyfan

Final Appraisal Recommendation

Advice No: 4415 – December 2015

Ivacaftor (Kalydeco®) 150 mg film-coated tablets

Submission by Vertex Pharmaceuticals (UK) Ltd

Recommendation of AWMSG

Ivacaftor (Kalydeco®) is recommended for use within NHS Wales for the treatment of cystic fibrosis (CF) in patients age 6 years and older who have one of the following gating (class III) mutations in the CF transmembrane conductance regulator (*CFTR)* gene: *G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N,* or *S549R*. This recommendation applies only in circumstances where the approved Wales Patient Access Scheme is utilised.

Additional note(s):

- AWMSG considered that ivacaftor (Kalydeco®) satisfies the AWMSG criteria for ultra orphan drug status.
- Clinicians should adhere to the WHSSC Clinical Access Policy for prescribing and monitoring of ivacaftor.
- Please refer to the Summary of Product Characteristics for the full licensed indication.

In reaching the above recommendation AWMSG has taken account of the appraisal documentation prepared by the AWMSG Secretariat (reference number 2294), 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 was ratified by Welsh Government and will be considered for review every three years.

Marketing authorisation holder on first issue	Vertex Pharmaceuticals (UK) Ltd
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