

lumacaftor-ivacaftor film-coated tablets, granules in sachet (Orkambi®)

Vertex Pharmaceuticals (Europe) Ltd.

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The Scottish Medicines Consortium (SMC) collaborated with the National Institute for Health and Care Excellence (NICE) and following review by the SMC Executive, SMC advises NHS boards and Area Drug and Therapeutics Committees (ADTCs) on the use of the above product in NHSScotland. The advice is as follows:

ADVICE: following SMC collaboration with NICE on TA988: *ivacaftor-tezacaftor-elexacaftor, tezacaftor-ivacaftor and lumacaftor-ivacaftor for treating cystic fibrosis.*

lumacaftor-ivacaftor (Orkambi®) is accepted for use within NHSScotland.

Indication under review: treatment of cystic fibrosis (CF) in patients aged 1 year and older (granules in sachet) or 6 years and older (film-coated tablets) who are homozygous for the *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene.

Full details of the assessment and recommendations can be found at <https://www.nice.org.uk/guidance/ta988>.

This advice applies only in the context of an approved NHSScotland Patient Access Scheme (PAS) arrangement delivering the cost-effectiveness results upon which the decision was based, or a PAS / list price that is equivalent or lower.

Advice context:

No part of this advice may be used without the whole of the advice being quoted in full.

This advice represents the view of the Scottish Medicines Consortium and was arrived at following collaboration with NICE. It is provided to inform the considerations of Area Drug and Therapeutics Committees and NHS boards in Scotland in determining medicines for local use or local formulary inclusion. This advice does not override the individual responsibility of health professionals to make decisions in the exercise of their clinical judgement in the circumstances of the individual patient, in consultation with the patient and/or guardian or carer.

Chair

Scottish Medicines Consortium