Vaughan Gething AC/AM
Ysgrifennydd y Cabinet dros Iechyd a Gwasanaethau Cymdeithasol
Cabinet Secretary for Health and Social Services

Llywodraeth Cymru
Welsh Government

Our ref VG/05162/17

David John Rowlands AM
Chair - Petitions Committee.
National Assembly for Wales
Cardiff Bay
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11 December 2017

Dear David,

Thank you for your letter of 21 November regarding Petition P-05-797 from Rhian Barrance regarding access to the cystic fibrosis medicine, Orkambi®.

We believe everyone should have access to cost-effective, evidence-based NHS treatment and care at all times to meet their clinical needs. This means ensuring decisions about the availability of treatment are based on evidence of effectiveness and the extent to which the benefits are in proportion to the cost charged by the manufacturer. To achieve this, we are guided by the recommendations of the National Institute for Health and Care Excellence (NICE) and the All-Wales Medicines Strategy Group (AWMSG).

NICE issued final guidance in July 2016 and did not recommend Orkambi® for routine use in the NHS in Wales or England. In December NICE re-issued its Technology Appraisal guidance under its "Do Not Do" guidance, emphasising this treatment should not be made routinely available. Whereas NICE frequently issue guidance which does not advocate using a specific medicine for a certain condition, it is far rarer for NICE to explicitly advise that a medicine should not be routinely used at all. The NICE independent appraisal committee found that when compared to the current standard of care, the clinical benefit offered was modest and comes at a considerable cost. The Scottish Medicines Consortium has also turned down this medicine for the same reason.

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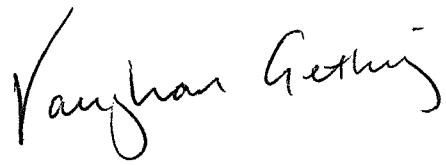
The All-Wales Medicines Strategy Group (AWMSG) has contacted the pharmaceutical company, Vertex Pharmaceuticals and has strongly encouraged them to make a submission to the AWMSG for appraisal. Whilst Vertex has agreed in principle to submit clinical data for appraisal by AWMSG, they have not committed to any firm date for doing so. If the manufacturer refuses to provide evidence about how well their medicine works, AWMSG cannot appraise it and cannot therefore issue a recommendation to make the medicine routinely available or not. However, discussions have commenced with Vertex on the most effective approaches to appraisal for the additional license extensions due to come on stream over the next few years. My officials will ensure the future appraisal of lumacaftor/ivacaftor (Orkambi®) is covered.

In the interim, the Welsh Health Specialised Services Committee (WHSSC) has agreed a patient access scheme with Vertex Pharmaceuticals and it is available in the Welsh NHS, where clinically appropriate.

Whilst compassionate use agreements offer treatment at no cost for a fixed period, NHS organisations must consider the implications of entering into such agreements including the clinical benefits for patients and the longer term cost implication for the NHS.

Where medicines such as Orkambi® are not routinely available within NHS Wales a clinician may apply for the medicine on behalf of their patient to an Individual Patient Funding Request (IPFR) panel in the appropriate health board. The clinician would need to source sufficient evidence to demonstrate the clinical and cost effectiveness of the proposed intervention.

Yours sincerely,



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