Vaughan Gething AC/AM
Y Gweinidog Iechyd a Gwasanaethau Cymdeithasol
Minister for Health and Social Services

Llywodraeth Cymru
Welsh Government

Our ref VG/05622/19

David John Rowlands AM
Chair
Petitions Committee
National Assembly for Wales
Cardiff Bay
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8 March 2019

Dear David,

Thank you for your letter of 21 February asking for clarity on an appropriate method of providing interim access to Orkambi® (lumacaftor/ivacaftor) to patients in Wales who may benefit from it.

I recently issued a Written Statement about Orkambi® which can be viewed at:

https://beta.gov.wales/written-statement-access-cystic-fibrosis-medicine-orkambir-lumacaftorivacaftor

As you are aware, in 2017 your Committee received evidence that Vertex Pharmaceuticals had gathered new evidence about Orkambi's clinical effectiveness since NICE's appraisal in 2016. Vertex agreed that the new evidence be appraised by the All Wales Medicines Strategy Group (AWMSG).

AWMSG contacted Vertex Pharmaceuticals about the new evidence again in November 2018 but the company has not yet submitted Orkambi® for re-appraisal. In December 2018, Vertex agreed to submit Orkambi® for re-appraisal and Symkevi® for appraisal by the Scottish Medicines Consortium. While the appraisals are being carried out, Vertex will provide these medicines at a discount. In order to prescribe them, clinicians will use the Peer Approved Clinical System (PACS) Tier 2 process, which is the Scottish equivalent of our Individual Patient Funding Request (IPFR) process.

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Where medicines such as Orkambi® are not routinely available within NHS Wales, a clinician may apply for the medicine on behalf of their patient through an IPFR. IPFRs are requests to a Health Board or to the Welsh Health Specialised Services Committee (WHSSC) to fund NHS healthcare for individual patients who fall outside the range of services and treatments that a Health Board has arranged to routinely provide or commission. An IPFR is an appropriate method for providing interim access to patients who would benefit from Orkambi® in Wales, whilst discussions over a full appraisal of the treatment continue.

It is for Vertex to decide whether it wishes to enter into a specific commercial arrangement for Orkambi® with the NHS in Wales, taking account of the uncertainties and anomalies identified by NICE. Any arrangement would require a clear and binding commitment to engage in a future health technology appraisal by NICE or AWMSG within a specified time (normally 12 months).

The new Voluntary Scheme for Branded Medicines Pricing and Access, which started on 1 January requires that any discount offered to one part of the UK be made available to all parts of the UK. This does not affect the Scottish proposal which was agreed just before the Voluntary Scheme started. Any new discount offered to the NHS in Wales would therefore be available to all other parts of the UK.

Without the evidence-based approach, which includes a clear set of criteria and independent clinical experts to appraise the clinical and cost-effectiveness of new medicines, the NHS would have no way of identifying medicines which are the most cost-effective and most clinically effective and would have no way of distinguishing between them.

The evidence-based approach in place in the UK is the safest method to ensure the most effective treatments for patients and the most effective use of NHS resources.

Yours sincerely,

Vaughan Gething AC/AM
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