

Cyfarwyddwr Cyffredinol Iechyd a Gwasanaethau Cymdeithasol/
Prif Weithredwr GIG Cymru
Grŵp Iechyd a Gwasanaethau Cymdeithasol

Director General Health and Social Services/
NHS Wales Chief Executive
Health and Social Services Group



Llywodraeth Cymru
Welsh Government

David Ramsden
Chief Executive
Cystic Fibrosis Trust
2nd Floor, One Aldgate
London
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Our Ref: AG/MM/SB

5 July 2018

Dear Mr Ramsden

Thank you for your letter of 26 June regarding the protests about the availability of Orkambi® as a treatment for cystic fibrosis.

We believe everyone should receive a comprehensive package of the best, 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.

Whilst I can appreciate your concerns regarding access to this treatment, in Wales, we remain guided by recommendations from the National Institute for Health and Care Excellence (NICE). NICE issued final guidance in July 2016 and it did not recommend this new medicine for routine use within the NHS. In December 2016, NICE re-issued its Technology Appraisal guidance under its 'Do Not Do' guidance, emphasising this treatment should not be made routinely available. 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 reasons.

Medicines in Wales can also be appraised by the All-Wales Medicines Strategy Group (AWMSG). However, following a negative decision by NICE, AWMSG would require information in addition to that considered by NICE. The All-Wales Therapeutics and Toxicology Centre (AWTTC) has contacted the pharmaceutical company, Vertex Pharmaceuticals, and strongly encouraged them to make a submission to AWMSG for

appraisal. To date, Vertex Pharmaceuticals has not indicated they will be making a submission to AWMSG.

Where medicines such as Orkambi[®] are not routinely available within NHS Wales then 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. I have enclosed a copy of the IPFR guidance which may be helpful to your constituent.

I hope you find this information useful.

Yours sincerely



Dr Andrew Goodall

Enc.