

**From the Permanent Secretary
and HSC Chief Executive**



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Dear MLA

Thank you for your letter of 10 November 2017 regarding access to lumacaftor and ivacaftor (Orkambi) for the treatment of Cystic Fibrosis.

The Department has a formal link with the National Institute for Health and Care Excellence (NICE) under which NICE Technology Appraisals are reviewed locally for their applicability in Northern Ireland. Where found to be applicable they are endorsed by the Department for implementation within Health and Social Care (HSC). NICE can only recommend that treatments be routinely funded within the NHS when there is clear evidence that they are both clinically effective and represent value for money.

NICE does not recommend the use of lumacaftor and ivacaftor (Orkambi) for treating cystic fibrosis and has published guidance to this effect (TA398). In August 2016 the Department endorsed TA398 as applicable in Northern Ireland. Therefore, Orkambi is not offered as a routinely commissioned treatment for cystic fibrosis in any of the UK jurisdictions.

NICE has confirmed that the position has not changed from the following statement provided in July 2017:

“Our appraisal of lumacaftor–ivacaftor (Orkambi) for treating cystic fibrosis will next be considered for review in July 2019. However, if new evidence becomes available that is likely to affect the current recommendations before then, or if the company puts forward a proposal that demonstrates their drug is cost-effective, we can consider whether it is appropriate to bring forward the review.

At this stage, we have not been informed of any evidence that could lead to a proposal to review the guidance, including any new proposals from the company.

We do understand how important a new treatment option would be for people with cystic fibrosis; but for the benefits it offers, the cost of Orkambi is currently too high. We can only recommend treatments be routinely funded on the NHS when we are certain they are both clinically effective and represent good value for money. If the

company is able to put forward a proposal that provides Orkambi at a cost-effective price, we would welcome it”.

The treatment of individual patients remains a clinical matter for health professionals in partnership with patients and their parents and carers. Such clinical decisions must be informed by the relevant medical and scientific evidence and guidance. In that context, it would be inappropriate for the Department to intervene in this process.

The use of Orkambi for treating cystic fibrosis will next be considered for review by NICE in July 2019. However, as the NICE statement above indicates, NICE will consider whether it is appropriate to bring forward the review and we will revert to that guidance. Companies are free to engage with NICE regarding the price and cost effectiveness of their drugs at any time.

The HSC Board met with Vertex to learn more about the potential and impact of the treatment. This was not a commercial negotiation and further meetings may be scheduled in the future.

The confidential contract between Vertex and the Health Service Executive (HSE) is a matter for that jurisdiction.

Yours sincerely

A handwritten signature in blue ink, appearing to read 'R. Pengelly', is centered on a white rectangular background.

RICHARD PENGELLY