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Dr Sarah Wollaston
Chair, Health and Social Care Committee
House of Commons
London
SW1A 0AA

16 January 2019

Dear Dr Wollaston,

On 30 November 2018, Vertex made its initial submission of documents to the Health and Social Care Committee regarding the Committee's inquiry into the availability of Orkambi on the NHS. Following this, we were invited to submit any further documents by 16 January 2019.

Enclosed please find the following documents provided to update the Committee on developments since our initial submission:

1. Letter from NICE to Vertex dated 13 December 2018, following Vertex's meeting with NICE on 30 November 2018
2. Letter from Vertex to NICE dated 16 January 2019
3. Letter from NHS England to Vertex dated 14 January 2019
4. Letter from Vertex to NHS England dated 16 January 2019

As you know, Vertex is committed to working with NICE and NHS England to provide access to our current and future treatments to patients with cystic fibrosis (CF). Since our 30 November 2018 submission, we have continued to engage with NICE. However, as long as NICE remains constrained by its current approach to the technology appraisal process, and, ultimately, by the budgetary limits set by the Department of Health and Social Care, it is unlikely that the parties will arrive at an outcome that grants access to our treatments for CF patients in England. Below we have highlighted just some of the aspects of NICE's current approach that do not appropriately factor in the benefits of innovative, disease-modifying medicines that treat rare, chronic conditions over a patient's lifetime.

In particular, we have been in discussions with NICE on the discount rate that it uses for appraisals, both generally and as applied to Vertex's medicines. NICE has not only departed from the current Treasury Green Book recommendation, it is claiming that an adjustment to the rate it is applying would require a formal consultation process. We are not aware of any legal obligation for this consultation process. Given the urgency to resolve differences on the price of our medications – and, ultimately, to bring our medicines to patients – it is particularly frustrating that this rate remains unchanged nine months since the updated Green Book recommendation was published.

Further, we are concerned that, despite initial constructive discussions on the topic, NICE has confirmed that its appraisal methodology will not take patent expiration into account in its assessment of the cost effectiveness of our treatments. As you know, medicines lose their market exclusivity and face generic competition when their patents expire, which causes their costs to the NHS to fall dramatically (typically 80-90%). Because NICE fails to recognize this commercial reality, it overstates the projected costs, which negatively impacts its assessment of the cost effectiveness of our medicines.

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In addition to the updates on discussions with NICE and NHS England, we wanted to make the Committee aware of recent developments in Scotland. Since Vertex's submission to the Committee at the end of November, we have been in further discussions with the Scottish Government as to how our medicines should be considered by the Scottish Medicines Consortium (SMC) for general availability to NHS patients in Scotland. The Scottish Government's engagement has facilitated constructive discussions, and we have now secured significant progress on both the SMC's appraisal process and making these products immediately available to patients. One particularly helpful factor in the discussion was the fact that the SMC appropriately leveraged the inherent flexibilities associated with its orphans medicines review process. Specifically, while NICE has only two options for appraisal (ultra orphan diseases and all other diseases), the SMC has a third option for orphan diseases, which can be used to more accurately assess the cost effectiveness of medicines like ours.

I hope this second submission is helpful. Vertex looks forward to meeting with the Committee to help with your inquiry and ultimately to bring our innovative CF treatments to patients in England.

Yours sincerely,

A handwritten signature in black ink, appearing to read 'Simon Lem', with a long horizontal flourish extending to the right.

Simon Lem
Vice President, Regional General Manager, Europe North