Dear Mr. Stewart,

Thank you for your letter of 14 January 2019. As you know, we have been working for years to find a solution that will make Orkambi and our other medicines for the treatment of cystic fibrosis (CF) available to English CF patients. We are committed to completing that journey, and we welcome your continued engagement.

We have long been concerned that NICE is constrained by its current approach to the technology appraisal process, which fails fully to capture the benefits or realistically assess the costs of life-extending medicines used to treat patients with rare diseases, like CF, throughout their lifetime.

That said, we agree with you that NICE can and should be flexible in applying its methodology to Vertex’s medicines for CF.

In particular, it is neither necessary nor appropriate in this case to default to NICE’s standard ICER threshold of £30,000/QALY.

We similarly agree with you that NICE can apply a discount rate of 1.5% in appropriate cases. This is clear in its current Methods Guide, although, in fact, the Methods Guide is out of date as the standard discount rate of 3.5% stated there does not reflect the latest edition of the Green Book (published in March 2018), which it is said to follow. As such, we have explained to NICE that it is appropriate to apply the updated policy to reflect that a default rate of 1.5% is recommended for health interventions. NICE’s consideration of the “exceptional” discount rate should also be recalibrated on this basis.

Additionally, the NICE evaluation process currently does not take into account that when medicines lose their market exclusivity after patent expiry, their costs to the NHS fall dramatically (typically by 80 - 90%). It is unrealistic to assume that a medicine would remain at its currently listed price over the entire model horizon, particularly when this can be upwards of 40 years. We have stated in meetings that
Vertex is prepared to apply a contracted reduction in medicine costs from a pre-defined number of years after reimbursement regardless of if there are generic products on the market or not. Adopting this approach would have a significant impact on the cost effectiveness of Vertex’s medicines.

After a productive in-person meeting on 30 November 2018, NICE’s letter to us on 13 December 2018 appeared to dismiss the prospect of any flexibility in their methodologies along these lines. Nevertheless, we remain actively engaged in seeking to identify meaningful flexibilities that NICE can adopt in order to reflect more accurately the cost effectiveness of these life-changing and life-extending treatments: see attached letter dated 16 January.

The flexibilities built into the Scottish Medicines Consortium’s (SMC) process for evaluating orphan medicines allows for a more realistic assessment of the cost effectiveness of medicines such as these. For example, the SMC has increased flexibility to accept a higher cost per ICER/QALY and apply ‘modifiers’ for medicines eligible for review under the SMC’s Patient and Clinician Engagement (PACE) process, which expressly takes account of views and evidence from patients and clinicians.

In Scotland — with the active support of the Scottish Government — we have been able to agree to a way forward that allows access for named patients now and a basis on which to submit our treatments for assessment by the SMC with a view to making them generally available as soon as possible.

Not least given our concerns about NICE’s current approach, we cannot agree to an arrangement in which we are bound to accept a price determined by NICE. NICE’s processes are not intended to be a substitute for effective negotiations between NHS England and manufacturers, nor should its processes be a barrier to achieving a good agreement at the earliest possible opportunity.

In Scotland, the leadership of the Scottish Government was instrumental in securing the significant progress we have described above. We would welcome the leadership of NHS England and the Department of Health and Social Care to help us make similar progress in England.

I look forward to hearing from you and Vertex stand ready to meet with you to progress this issue further. As you are aware, the Health and Social Care committee has requested copies of communications between Vertex, NICE and NHSE as part of the ongoing inquiry, and so we will share a copy of this letter with Dr Sarah Wollaston, Chair of the Health & Social Care Committee.

Yours sincerely,

[Signature]

Dr Jeff Leiden
Chief Executive Officer, President