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PRIVATE AND CONFIDENTIAL

BY EMAIL
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NICE's methods for technology appraisals

Thank you for your recent correspondence and your time at the meetings of 4 October 2018 and 30 November 2018 where we discussed NICE's methods for the appraisal of innovative medicines used to treat patients with rare diseases, such as our treatments for cystic fibrosis (CF).

We welcomed the flexibility and desire to find a creative solution that you expressed in those meetings, and understood you would go away and discuss our suggestions and approach with other members of the NICE team and NHSE. We understood there was agreement that our requests were reasonable especially in the context of appraising innovative medicines used to treat patients with rare diseases.

We were therefore surprised and disappointed by the position set out in your subsequent letter of 13 December 2018. From this response, it seems clear that a NICE assessment of our existing and future medicines would not produce a materially different outcome than the outcome for Orkambi.

We remain committed to finding a solution that secures access to the life-changing treatments that CF patients in England deserve and hope that we will be able to find a path forward. In that spirit, we are writing to you to address some of the specific points made in your letter.

Discount rate

Thank you for your clarification in relation to the discount rate used in NICE appraisals in general and, in particular, in relation to the appraisal of our treatments. As you know, there is no statutory requirement for NICE to apply a particular discount rate under the legislation that governs the exercise of its functions.

The NICE methods guide, to which your letter refers, states that, for the reference case, NICE usually considers it appropriate "*to discount costs and health effects at*

the same annual rate of 3.5%, based on the recommendations of the UK Treasury for the discounting of costs" (see para 5.6, emphasis added). As your letter states, a different discount rate may be applied in non-reference cases.

The recommendations of the UK Treasury upon which your guide is (rightly) predicated have changed. As your letter acknowledges, the updated Green Book guidance recommends a 1.5% rate for health interventions. We do not understand (and your letter does not explain) the basis on which NICE has decided now to depart from the Green Book recommendation.

Your letter states that, before any change is made to the discount rate you apply, you must "*subject any proposal to do so to formal consultation*." We are not aware of any legal obligation on you to consult in these circumstances, in particular where any change would simply be to ensure that your guide continues to reflect the Government policy on which it is expressly based.

However, if consultation were necessary on this point, then it is highly regrettable that it has not been undertaken in the nine months since the updated Green Book was published (in March 2018), and we would urge you to implement the updated policy as soon as possible 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.

Contracted discount upon patent expiry

NICE 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. As discussed, 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.

Engaging with NICE in the appraisal of tezacaftor-ivacaftor and the review of lumacaftor-ivacaftor

As we have discussed with you previously, we are committed to working with NICE to find a route to providing eligible patients in England with access to our treatments. However, we remain concerned that NICE is constrained by its current approach to the current technology appraisal process, which fails fully to capture the benefits or realistically to assess the costs of life-extending medicines used to treat patients with rare diseases, like CF, throughout their lifetime.

The discount rate applied by NICE and accepting a contracted discount in price after a certain number of years are two of a number of aspects of NICE's methodology that constrain the appropriate evaluation of our medicines. These are aspects of an evaluation that NICE can change, and we urge you now to address them.

Doing so would be a clear signal that NICE is willing and able to engage with the shared challenge that we face to deliver these treatments to the people that need them, and it would be a material step towards a workable approach.

Progress in Scotland

As you may be aware the Scottish Medicines Consortium (SMC) has flexibilities built into their process for evaluating orphan medicines. For example the SMC has increased flexibility to accept 'modifiers' for medicines eligible for review under the SMC's Patient and Clinician Engagement (PACE) process. We have seen the potential of such an approach in Scotland and have been able to agree 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.

We are ready to meet again or arrange a call to discuss further. I also wanted to let you know that I will share a copy of this letter with Dr Sarah Wollaston, Chair of the Health & Social Care Committee of the House of Commons, as the Committee has requested copies of communications between Vertex, NICE and NHSE as part of the ongoing inquiry.

I look forward to hearing from you.

Yours sincerely,

Simon Lem
Vice President and General Manager Northern Europe Region