



Health and Social Care Committee

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From Dr Sarah Wollaston MP, Chair

Rt hon Matt Hancock MP

Secretary of State for Health and Social Care

8 March 2019

Dear Matt

Availability of Orkambi on the NHS

We understand that you will be meeting Dr Jeff Leiden and other representatives of Vertex Pharmaceuticals shortly to discuss the availability of Orkambi, and other drugs for the treatment of cystic fibrosis, on the NHS. You will be aware that the Health and Social Care Committee has been considering this matter, and that yesterday we took evidence on the issues leading to the current impasse. This letter is to inform you of our conclusions having heard from clinician and patient representatives, NICE and NHS England, and Dr Leiden himself.

Our starting-point is that the best interests of patients should be paramount in the arrangements for access to Orkambi, and other treatments in the pipeline, including Symkevi and the "triple therapies" which we understand are in phase 3 development. We heard evidence both in writing from the cystic fibrosis community and in person from Oli Rayner, a CF patient, and Dr Caroline Elston, a leading clinician, of the benefits of these medicines to those living with cystic fibrosis which they do not feel have been fully captured by the evidence which NICE has considered so far. Patients are being denied access whilst the arguments rage about the returns on Vertex's investments, NICE's appraisal processes, and NHS England's understandable responsibility for managing a finite budget and the needs of all patients. We would like to see interim access agreed whilst the wider issues are further debated, as has happened elsewhere.

We were told of a significant gap between the wider benefit which patients and clinicians are describing from the drugs, and Vertex's clinical evidence which has been assessed by NICE. That gap is apparent not only in NICE's appraisal of the product, but in the views of other appraisal bodies, such as in Canada, who have not judged Orkambi to be sufficiently cost effective.

Vertex is arguing that the reason for that gap is that NICE's processes are outdated and are not suitable for the appraisal of new therapies such as the ones it has been developing for CF. The Committee recognises that NICE is an internationally respected body which has provided a model for similar appraisal bodies around the world. Vertex

needs to be able to provide evidence of the efficacy and value of its therapies on the same basis as expected of any other company and the Committee is concerned that it has stopped engaging with NICE. Furthermore, the NHS should not be expected to face costs for these drugs that simply cannot be justified by their cost benefit. In the context of finite resources, to do so would be to deny other NHS patients the benefit of effective therapies.

Vertex argues that it cannot drop its price because to do so would hinder its ability to continue to undertake research and development leading to further drug discoveries. Whilst we recognise the importance of continuing research and development in the pharmaceutical industry, we do not consider that an acceptable ground for paying far more for a drug than can be justified by its cost effectiveness. We also note the benefit obtained by Vertex from philanthropic funding bodies during the development of their products. NICE's processes are well-established and Vertex will have known, when it made investment decisions, about the approach NICE and the NHS would take to the reimbursement of its therapies. Vertex appears to have decided on the pricing of its therapies on the basis of the return it wants to make, rather than the value which they bring. NHS England is right to continue to take the wider patient population for whom it is responsible into account.

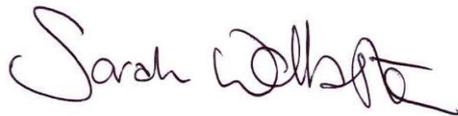
In order to reach an acceptable solution to this situation, however, flexibility needs to be shown by both sides. Vertex should re-engage with NICE on the value of its therapies. NICE and NHS England, on the other hand, need to enable Vertex to provide evidence of the benefit which cystic fibrosis clinicians, patients and their families have told us they have seen where these therapies have been made available. And access to these drugs needs to be made available as soon as possible with both sides prepared to be flexible on renegotiating as further evidence emerges over the coming years. The NHS is ideally placed to be able to provide ongoing evidence and the CF community is clearly keen to help with this.

We therefore urge you to encourage Vertex, NHS England and NICE to explore urgently how access to Vertex's drugs for the treatment of cystic fibrosis can be made available as soon as possible, alongside a plan for collecting evidence on the effectiveness of the drugs which will enable NICE to make an assessment of its value which takes into account the real-world experiences of patients and their families. We invite you to urge Vertex, at your meeting on Monday, to accept the necessity of demonstrating the worth of its therapies in accordance with the internationally-respected processes run by NICE; but in return, to assure Vertex that you will encourage NHS England and NICE to ensure that it is able to do so with high quality data which fully capture the evidence. If successful—and if Vertex cooperates with NICE in the collection of high-quality evidence—this can facilitate a flexible funding arrangement over time whereby NHS payments to Vertex reflect the evidence of clinical benefit in NHS patients. We accept that finding a mutually acceptable way through the current impasse will not be easy: it will require both sides to show even greater flexibility than they have done so far. In particular, it will require Vertex to re-engage with NICE processes; and it will require NHS England to reconsider its indication that its July 2018 proposal was its "best and final offer". But all sides were keen to assure us that they remain committed to finding a solution. We, and I am sure you, will be looking to them to demonstrate that commitment, to us and to the cystic fibrosis community, by returning to the table.

In the longer term, if Vertex cannot be persuaded to re-engage meaningfully in NICE processes, we suggest that you might want to explore the possibility of referring the company to the Competition and Markets Authority for what appears to us to be the exploitation of a monopoly position in the supply of drugs for the treatment of cystic fibrosis. We note also Steve Brine's indication in a recent debate on this issue that the Government has not ruled out the use of a Crown Use licence although we note that this would be complicated and would not result in rapid access to these drugs.

We are copying this letter to Dr Leiden, to John Stewart and Prof Powis at NHS England, and to Sir Andrew Dillon and Meindert Boysen at NICE. We urge all five of them to continue to keep at the forefront of their mind the needs of cystic fibrosis patients, and to do all they can to ensure that these therapies can be provided to them at a price which does not disadvantage the well-being of the many others who also rely on the NHS for their medical care.

Yours sincerely,

A handwritten signature in black ink, appearing to read 'Sarah Wollaston', with a stylized flourish at the end.

Dr Sarah Wollaston MP
Chair of the Committee