Cystic Fibrosis beatable

George Freeman MP
Minister for Life Sciences
Department of Health/BIS
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Dear George,

NICE have today published their provisional guidance for lumacaftor-ivacaftor therapy (Orkambi®).

NICE's Appraisal Consultation Document (ACD) describes the therapy as both effective and important in the management of cystic fibrosis but has nevertheless decided to not recommend the therapy for use in England.

I can only imagine that you share my deep frustration and disappointment in an outcome that has been as predictable as it is distressing for thousands of families.

We have described our concerns that the Single Technology Appraisal (STA) process has been inappropriate for considering this highly innovative, first-in-class therapy at each juncture since our engagement with it began in May last year. It gives me no joy whatsoever to see our predictions play out over the course of the process.

The ACD demonstrates the serious, dedicated approach that NICE takes as a matter of course but, heartbreakingly, desperately exposes the inflexibility of a system unable to cope with the challenges of assessing rare-disease medicines.

Specifically, the most exciting aspects of this therapy – the outcomes which people with cystic fibrosis, their families and clinicians all recognise as the most important – reductions in acute ill-health episodes, the need for hospitalisation and long-term health preservation, are underscored by uncertainty that clinical trials will never be able to address.

This will fatally undermine NICE's ability to reach an access agreement with Vertex Pharmaceuticals, the producers of the medicine.

This is perhaps the most disappointing aspect of the ACD. We appreciate that the NHS has a duty of care to all patients and this means difficult decisions must be taken on areas of investment. However, when the benefits of an intervention appear poorly understood, the question of determining value is rendered useless.

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This is a challenge that you have publicly recognised in the assessment of many new interventions and one that we have discussed, both in person and in correspondence from June last year and January this year, this therapy will be subject to.

The multi-national trials that assessed lumacaftor-ivacaftor therapy (Orkambi®) were the largest ever conducted for a new therapy in cystic fibrosis, recruiting over 1100 people with cystic fibrosis. If our institutions cannot confidently predict the value of a new intervention in such circumstances, it is our moral imperative to forge innovative pathways to ensure that those who can benefit do – and time is of the essence.

We stand ready, as you know we have done for more than 18 months, to broker an innovative solution to this fundamental challenge, harnessing the power of the UK Cystic Fibrosis Registry and our integrated network of specialist cystic fibrosis care centres to deliver a programme of evaluation that will address uncertainty in the evidence considered by NICE with real-world data.

The Cystic Fibrosis Trust propose an interim arrangement between the company and the NHS that would allow access coupled with a detailed examination of impact using the UK CF Registry. Our solution is created out of collaborative working with people with the condition, their families and specialist cystic fibrosis clinicians. We attach the principles that we have agreed upon, and believe it meets the challenge set by your Department's Accelerated Access review.

Just yesterday, the review published its supporting evidence for the final report, citing the UK CF Registry as an exemplar of potential sources of real-world data.

Now, our proposal requires effective collaboration between Vertex and the NHS and we are working with both. We are pleased that, having reviewed our proposal in detail, the company has indicated that it is willing to enter into discussions on this basis.

We now call on you to continue to help us to bring the Government, NICE and NHS England to the table to discuss this proposal at the earliest opportunity.

Please meet with us as a matter of urgency to get this right.

Yours sincerely

Ed Owen
Chief Executive
Cystic Fibrosis Trust

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