Cystic
Fibrosis a fight we must win

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Dear Sirs,

Letter to Vertex and NHS England – Orkambi

We are disappointed but not surprised by today's decision by NICE not to recommend the transformational lumacaftor/ivacaftor combination cystic fibrosis treatment Orkambi for use in England by the NHS.

It is clear that the accepted clinical outcomes produced by Orkambi are those 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.

What appears to be at issue is the unpredictability of long-term value and impact and therefore Orkambi's cost-effectiveness. Since NICE undertook its appraisal, new data published last month from the rollover study and based on 96 weeks of analysis suggests Orkambi almost halves the rate of decline in lung function in people with CF over a two-year period, potentially adding years to life. I have enclosed this data for reference.

The multi-national trials that assessed Orkambi were the largest ever conducted for a new therapy in cystic fibrosis, recruiting over 1,100 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.

This is why the Cystic Fibrosis Trust has proposed an innovative solution, with full clinical backing, 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.

With the NICE process complete, we call on Vertex and NHS England to begin immediate negotiations to agree a cost-effective solution based on our proposal. We stand ready to help in any way and are looking for progress by the end of July at the latest.

I look forward to hearing from both of you with your respective commitments to making this happen.

Yours sincerely,

Ed Owen

Ed Owen
Chief Executive