Cystic Fibrosis why we're here

Shona Robison
Cabinet Secretary for Health, Wellbeing and Sport
T4.02
The Scottish Parliament
Edinburgh
EH99 1SP

Dear Ms Robison,

The SMC have today published their guidance for two ground-breaking cystic fibrosis therapies: lumacaftor-ivacaftor therapy (Orkambi®) and the use of ivacaftor (Kalydeco®) for infants aged two to five years old.

The SMC has been unable to recommend either therapy for use in NHS Scotland. I can only imagine that you share my deep frustration and disappointment in an outcome that will cause significant distress for hundreds of Scottish families.

Kalydeco® is already available in Scotland for people with cystic fibrosis who carry the rare 'Celtic gene' mutation, G551D, and its impact has been transformational. It is inconceivable that young children who would be able to significantly reduce and avoid years of disease progression will be denied.

It flies in the face of the principle of prevention over rescue therapy and leaves children with a debilitating life-long condition playing a waiting game at a crucial stage of their lives.

I hope that you will act with utmost urgency to ensure that eligible children in Scotland will get access to this medicine as soon as possible.

With regard to Orkambi®, 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 uncertainties that traditional clinical trials will never be able to address.

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.

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 a challenge that you have publicly recognised in the assessment of many new interventions.

We stand ready 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 the SMC with real-world data.

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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.

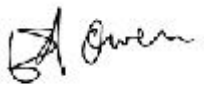
Our solution is created out of collaborative working with people with the condition, their families and specialist cystic fibrosis clinicians. We attach the principles that we have agreed upon.

In April, the UK Government's Accelerated Access Review – aligned with the Scottish Government's independent review of the way drugs are assessed for use, led by Dr Brian Montgomery – 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, 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 Scottish Government, SMC and NHS Scotland to the table to discuss these issues at the earliest opportunity. Please meet with us as a matter of urgency to get this right.



Ed Owen
Chief Executive, Cystic Fibrosis Trust