Cystic Fibrosis beatable

The Lord Prior of Brampton
Department of Health
Richmond House
79 Whitehall
London
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November 15 2016

Dear Lord Prior,

Cystic fibrosis, ORKAMBI and the Accelerated Access Review

There are lots of encouraging recommendations in the Accelerated Access Review (the Review) for the many life-changing cystic fibrosis treatments under development.

However, we are concerned that the focus on future treatments may detract from efforts to make ORKAMBI available to the 2,789 people that so desperately need it. ORKAMBI gained its European marketing authorisation a year ago and would benefit greatly from flexible pricing arrangements, confidential commercial agreements, and the capture and analysis of real-world outcomes – as outlined in the Review.

ORKAMBI is a perfect candidate drug for such an approach, especially given the power of the UK CF Registry to capture data to support a managed access scheme. This proposal is all the more important in the context of new 96-week data published this week which shows that ORKAMBI slows the decline in lung function – important as the main indicator of mortality risk - by 42%. This data was unavailable to NICE and is comparable to the efficacy of KALYDECO (47%) a drug which is already being used to treat hundreds of people with one of nine specific cystic fibrosis-causing mutations. A situation in which a life-changing precision medicine is available to one particular genetic class, but not others is, in our opinion, untenable.

ORKAMBI meets the criteria referred to in the Review for ‘transformative’ designation. We understand that Vertex is willing to embrace our solution and is keen to agree a commercial arrangement with NHS England, but negotiations appear to have stalled because none of the flexibility recommended in the Review is currently available.

The people waiting to access ORKAMBI do not have the luxury of time. With every day that passes more irreversible damage is inflicted on their lungs and other organs. The delay in getting this treatment to people will mean that more will require transplants and some will die

earlier. People cannot wait for the development of a Strategic Commercial Unit to consider innovative commercial arrangements.

Please help us. I would be extremely grateful if you could seek guidance from NICE and NHS England on what immediate action can be taken to reach agreement with Vertex so Orkambi is available to those who need it as soon as possible. We are also

I enclose information on the 96-week ORKAMBI study which demonstrates the potential of the drug to slow the progression of the condition and transform the clinical pathway.

We look forward to hearing from you.

Yours sincerely,

Darren O'Keefe
Public Affairs Manager