

# Cystic Fibrosis a fight we must win

David J Rowlands AM  
Chair, Petitions Committee  
National Assembly for Wales  
Cardiff Bay  
Cardiff,  
CF99 1NA

01 October 2018

Dear Mr Rowlands,

We are grateful for the continuing efforts of the Petitions Committee of the Welsh Assembly in exploring access to the most advanced cystic fibrosis medicines in Wales, through communication with Vertex Pharmaceuticals and the Cabinet Secretary. I know that you appreciate that urgency is vital.

The petition 'Ensure access to the cystic fibrosis medicine, Orkambi, as a matter of urgency' was handed in on 10 January 2018 and people with cystic fibrosis in Wales have already waited nearly three years for access to the drug since its European license in November 2015, and subsequent appraisal by the National Institute for Health and Care Excellence (NICE). During this time the cystic fibrosis community have experienced bitter disappointment, deterioration and, for some, death.

Disease-modifying therapies, such as Orkambi, which tackle the root cause of cystic fibrosis, are offering hope to our community. There are 433 with cystic fibrosis in Wales, around half of whom could stand to benefit from Orkambi.

As we celebrate these advances in cystic fibrosis treatment, Orkambi is still not readily available to those patients who so desperately need it.

The Trust calls on all parties involved to accept their responsibility in making sure these treatments are made available to Welsh people with cystic fibrosis, and that a resolution is agreed that will enable this to happen as soon as possible.

The Trust urges the Welsh Government to consider immediate access through an interim arrangement so that people with cystic fibrosis do not continue to be made to suffer in an ongoing impasse. The Trust reiterates the importance of the UK CF Registry in bridging the uncertainty gaps associated with these medicines while providing real world data to monitor the clinical impact of the drug. Please find our principles of managed access to new cystic fibrosis therapies paper attached.

Yours sincerely,



David Ramsden  
Chief Executive