Cystic Fibrosis our focus

e-Petition: Ensure access to the cystic fibrosis medicine, Orkambi, as a matter of urgency

1. Orkambi is only one of a new class of medicines for cystic fibrosis. These new precision medicines work in a completely different way to today's treatments. Today's treatments are indiscrete, aggressive, and take between three and five hours out of each and every day. Tomorrow's treatments could be different. Accessing Orkambi a critically important next step in revolutionising cystic fibrosis care into a genuinely stratified and personalised model. By 2020, is it estimated that 90% of cystic fibrosis patients could be eligible for new precision medicines. Yet, we have been waiting for two and a half years for this next step. Patients still cannot access Orkambi. We have been waiting whilst people's health, life expectancy and quality of life are declining. Last year, the median age of death for people with cystic fibrosis was 31 years.

2. There has been no process and no sign of progress for the hundreds of people with CF who could benefit in Wales. To date no-one has been held accountable for securing access to these new treatments in Wales since NICE appraised Orkambi in July 2016. The correspondence from the Cabinet Secretary states that the manufacturer of Orkambi, Vertex has, as yet, failed to engage with the AWMSG to the detriment of patients. We need our representatives to publicly hold Vertex to account if this is true. We need debate in a public forum to show that the Welsh government is actively driving forward a deal on behalf of Welsh citizens.

3. This month Vertex submitted new Patient Access Schemes to NHS England and NHS Scotland for access to precision medicines for cystic fibrosis. In addition to Orkambi, it could include access to Symdeko, the next precision medicine

for cystic fibrosis – approved by the FDA two weeks ago and due for EMA marketing authorisation this month. We are informed the approach has secured access in the Republic of Ireland and the Netherlands. However, we have heard nothing as to whether this approach is being considered in Wales, or if Vertex have made this offer for patients in Wales.

4. There is a strong mandate for this petition. In addition to the 5715 people who signed this e-petition before it closed in December 2017, over 12,000 people from Wales have signed a separate community led petition to secure UK access to precision cystic fibrosis medicines. Westminster MPs are debating this issue on Monday 19 March. However, it is the Welsh government who can secure access for the patients in Wales represented by those 12,000 signatories. Only Wales can negotiate and secure a Welsh solution for Welsh patients. We need public debate about how we will transform cystic fibrosis care in the future.

Cystic Fibrosis

Cystic fibrosis is a life-shortening genetic condition that affects over 10,400 people in the UK. The condition primarily affects the lungs and digestive system. The condition requires a huge burden of daily treatments including nebulisers, physiotherapy and pills to help control symptoms. Children and adults with cystic fibrosis spent on average 3-5 hours on treatment every day.

Last year, half of all people who died with cystic fibrosis were **under the age of 31.**

Precision Medicines

Standard cystic fibrosis treatment aims to lessen symptoms and complications. However, progressive damage still occurs, meaning symptoms and complications increase with age.

Precision medicines tackle the underlying cause of cystic fibrosis rather than just managing the symptoms. Orkambi has been shown to slow decline in lung function

by 42% and cut the number of infections requiring hospitalisation by 61%. This gives people more control over their lives and greater quality of life.

Portfolio Approach

This month Vertex submitted a new Patient Access Scheme to NHS England and NHS Scotland.

The offer is described by Vertex as a 'portfolio approach'. We are informed the approach is similar to that adopted in the Republic of Ireland and the Netherlands. Vertex states this approach would allow access to existing and new Vertex medicines within a set budget for the NHS.

Using the UK CF Registry to deliver a fair deal

The UK CF Registry is sponsored and managed by the Cystic Fibrosis Trust. The UK CF Registry offers population level coverage for people with cystic fibrosis in the UK. Anonymised, aggregated data from the registry are used as the evidence base for commissioning NHS care and post-marketing pharmacovigilance for the European Medicines Agency (EMA). [1]

In reimbursement decisions, the UK CF Registry could offer real world evidence of efficacy using observational comparative cohort models. The UK government could access this data, using it to inform a fair deal for cystic fibrosis medicines now and in the future.

[1] Data Resource Profile: The UK Cystic Fibrosis Registry, Taylor-Robinson D, University of Liverpool. 2017.