Petition consideration briefing:

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

We call on the Welsh government to agree a sustainable solution for cystic fibrosis medicines today and into the future.

1. Cystic fibrosis

Cystic fibrosis is a life-shortening genetic condition that affects over 10,400 people in the UK, including 398 people in Wales. Last year, half of all people who died with cystic fibrosis were under the age of 31.[1]

2. Precision medicine

Traditional treatments for cystic fibrosis aim to lessen symptoms and complications. However, progressive damage still occurs, meaning that these symptoms and complications will inevitably increase with age.

New precision medicines target the dysfunctional protein that causes cystic fibrosis. They target specific mutations and have the potential to preserve or restore lung function, slow decline and improve life expectancy.

The first two precision medicines are Ivacaftor (effective for under 10% of people with cystic fibrosis) and Orkambi (effective for around half of people with cystic fibrosis in Wales).[2] Many further precision medicines are being developed. Within five years, around 90% of people with cystic fibrosis could be treated with new drugs[3] that transform cystic fibrosis from a condition that is life threatening to one you that you live with and manage.

People with cystic fibrosis in Wales have already faced significant delays in accessing new precision treatments and are increasingly concerned that they will not get to access these life-changing medicines.

3. Orkambi

Orkambi is a precision medicine that tackles the underlying cause of cystic fibrosis rather than just managing the symptoms. The drug specifically targets the genotype with homozygous dF508 mutations which affects around 189 people in Wales.

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[1] UK CF Registry Report 2016, Cystic Fibrosis Trust, 2017.
[2] UK CF Registry Report 2016, Cystic Fibrosis Trust, 2017.
[3] http://www.businesswire.com/news/home/20170718006344/en/

Orkambi has been shown to slow decline in lung function by 42%[4] and cut the number of infections requiring hospitalisation by 61%.[5] This gives people more control over their lives and greater quality of life.

Yet people with cystic fibrosis in Wales cannot access Orkambi. It has been two years since the licence of Orkambi. Orkambi is available to all eligible patients in Austria, Denmark, France, Germany, Luxembourg, the Netherlands, Italy, Ireland, Greece, and the United States.

In July 2016, NICE announced they were unable to recommend the drug due to its cost, despite acknowledging the drug is "important and effective".[6] Since then, we have relentlessly called for negotiations and a fair, sustainable pricing deal for Orkambi. However, there has been no progress. People with cystic fibrosis are still waiting whilst their health and quality of life declines.

4. Using the UK CF Registry to deliver a fair deal

We urge the assembly to consider how the UK CF Registry could form part of a robust and fair access scheme bridging the uncertainty gap associated with these medicines.

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).[7] This means that it is possible to keep track of the effectiveness of Orkambi and other precision medicines to aid long-term monitoring.

In reimbursement decisions, the UK CF Registry could offer real world evidence of efficacy using observational comparative cohort models. The UK CF Registry already uses these models to assess the real-world efficacy of Ivacaftor in a study for the European Medicines Agency (EMA).[8]

This available data could inform a fair deal that secures access to transformational medicines for people with cystic fibrosis – now and in the future.

5. Response to the Cabinet Secretary for Health and Social Care

In his letter Health Minister Vaughan Gething states:

'The NICE independent appraisal committee found that when compared to the current standard of care, the clinical benefit offered was modest and comes at a considerable cost'.

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[4] http://www.businesswire.com/news/home/20161027005917/en/
[5] https://www.nice.org.uk/guidance/ta398/documents/appraisal-consultation-document
[6] https://www.nice.org.uk/guidance/ta398/documents/html-content-2
[7] Data Resource Profile: The UK Cystic Fibrosis Registry, Taylor-Robinson D, University of Liverpool. 2017.
[8] http://www.ema.europa.eu/docs/en_GB/document_library/EPAR_-_Assessment_Report_-_Variation/human/002494/WC500198918.pdf

However, the data available at the time of NICE appraisal was from two short-term studies of 24 weeks. We now have long-term 96-week study data from which Orkambi has been found to slow decline in lung function by 42% and cut the number of infections requiring hospitalisation by 61%. These are significant outcomes not previously considered in the original appraisal.

Mr Gething also refers to a 'patient access scheme with Vertex Pharmaceuticals' which is 'available in the Welsh NHS, where clinically appropriate.' Whilst we welcome compassionate use of Orkambi in Wales, only the sickest patients are eligible to be considered when significant damage has already been done. We need a system which gives everyone with cystic fibrosis the earliest chance to access these medicines as soon as they are available and before irreparable lung damage has occurred.

The Cystic Fibrosis Trust is aware that meetings between Vertex and NHS Wales have been taking place since June and are ongoing to discuss a possible deal around a portfolio approach, which could look to incorporate Orkambi and potentially future treatments for CF. We welcome these discussions but emphasise that a resolution needs to be reached urgently.

Case study one- Rhian Barrance, 31 from Cardiff

One of my closest friends, Jen, gave birth to a beautiful baby boy called Lorcán. Everyone who meets him falls in love with him immediately – he is bright, inquisitive and has Jen's gorgeous big brown eyes. But Lorcán needs more help and support than most children. Ten days after he was born he was diagnosed with cystic fibrosis. His extraordinary parents have launched themselves into doing everything they could to keep him as healthy and happy as they can.

I knew nothing about cystic fibrosis at the time, and spent a lot of time reading about it to figure out how I could best help and support Jen. When I found out about Orkambi, I threw myself into campaigning and it was then that I realised that there was something I could do – I could fight for the potentially life-changing treatments which target cystic fibrosis at its root cause to be made available to everyone in Wales who could benefit from them. This is why I started the e-petition to push for a debate in the Welsh Assembly to be considered.

Those who are currently eligible need access to it now as their health is deteriorating by the day. Lorcán and other children need Orkambi to be available to them as soon as they are old enough to take it. The National Assembly for Wales has made a strong commitment to children's rights through the Rights of Children and Young Persons (Wales) Measure (2011). One of the guiding principles of the United Nations Convention on the Rights of the Child (UNCRC) is the right to life, survival and development (article 6). Wales is often at the forefront in matters of children's rights, and providing children with a drug that can limit the lung damage caused by cystic fibrosis is a key part of realising the rights of children with the condition.

Ever since Lorcán was diagnosed with cystic fibrosis, he has faced a daily regime of treatment to manage his lung decline. These treatments do not address the underlying causes of the disease, and Lorcán's lung capacity will continue to decline despite the burden of constant drugs and physiotherapy. It is very difficult to accept that there is a treatment which could radically reduce the lung decline of people with this cruel condition, that is not available in Wales. It is particularly difficult to accept when this drug is available in many other countries, but this success abroad is an exciting prospect which gives us hope and makes me more determined than ever to keep up this fight.

I urge the petitions committee to enable a debate to go ahead in the assembly to highlight the plight of those adults and children who cannot access this drug and to call for a solution that will allow urgent access to this life-changing drug in Wales.

Case study two- Beth Clarke, 36 from Cardiff

I'm 36 years old and want to look forward to my future with my husband, but a huge chunk of each day is taken up by a regime that I must do to stay well, reminding me that I have a life-shortening genetic condition-cystic fibrosis (CF). My morning schedule of inhaled nebulisers and physiotherapy takes two-and-a-half hours in a grueling routine to stay alive, all before I can reach for a cup of tea. Using a machine to exercise my airways enables me to take deep breaths that my body wouldn't otherwise allow.

Coughing and spluttering my way through the day, I take upwards of 40 tablets and try to fit in half an hour of exercise to maintain as good health as I can. Despite this vigorous set of daily tasks, it is clear that I am battling with the symptoms-chest pain, shortness of breath, fatigue and not to mention the sickness that can accompany courses of intravenous antibiotics every six weeks. I'm doing my best and my family and I stay as positive and hopeful as we can- a mantra my parents have instilled in me- but I'm not tackling my cystic fibrosis at its core in a way that precision medicines like Orkambi would be able to do. My efforts are sometimes just not enough and my health can quickly deteriorate.

A treatment that could dramatically change my life for the better is something I have always talked and dreamed about with my parents and yet now that Orkambi is within touching distance I have to remind myself not to get my hopes up too much until it is available in Wales. It is incredibly cruel for all of us, knowing that Orkambi is available in countries across the world when our fight continues.

I urge the Welsh Assembly to debate this issue. I don't want my family and friends to be faced with another battle for medicines that could so easily change my life and the lives of others with cystic fibrosis. Please end the wait and give us hope again.