Y Gwasanaeth Ymchwil | Research Service

# Briefing for the Petitions Committee

Petition number: P-05-0797

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

We call on the National Assembly for Wales to call for a resolution to ongoing negotiations between NHS Wales, the All Wales Medicines Strategy Group, the Welsh Health and Specialised Services Committee and Vertex Pharmaceuticals regarding access to the cystic fibrosis medicine, Orkambi, as a matter of the utmost urgency.

418 people in Wales have cystic fibrosis (CF). CF is a life-shortening, inherited disorder. The median age at death for a person with CF in 2016 was just 31 years of age. CF is caused by mutations in the CFTR gene which result in the build-up of thick, sticky mucus in the lungs and other organs. Gradually, this build up causes chronic lung infections and progressive lung damage. The treatment burden for a person with CF is high and daily life can be a struggle.

Orkambi is a precision medicine that 40% of people in the UK with CF could benefit from. While conventional CF treatments target the symptoms, precision medicines tackle the underlying genetic mutations that cause the condition. Though Orkambi is not a cure, it has been found to slow decline in lung function – the most common cause of death for people with CF – by 42%.

In July 2016, the National Institute of Clinical Excellence (NICE) recognised Orkambi as an 'important treatment.' They were, however, unable to recommend the drug for use within the NHS on grounds of cost effectiveness and a lack of long-term data.

In June 2017, the Cystic Fibrosis Trust organised a day of national protest at the Senedd, Stormont, Holyrood, Downing Street and online to demand an end to the deadlock. Since the protests, the Welsh Health and Specialised Services Committee (WHSSC) have presented the All Wales Medicines Strategy Group (AWMSG) with the portfolio approach developed by the drug's manufacturer, Vertex Pharmaceuticals.

We call on the National Assembly for Wales to call for a resolution to these ongoing negotiations between NHS Wales, the AWMSG, WHSSC and Vertex Pharmaceuticals as a matter of the utmost urgency. It is essential that a fair and sustainable method of reimbursement is found for Orkambi and for the exciting pipeline of future treatments.



RS Ref: 17/5057

People in Wales have been waiting too long for this transformative drug. They deserve better.

## Policy background

Orkambi (lumacaftor-ivacaftor) is a medicine for the treatment of cystic fibrosis in patients aged 12 years and older who have a specific gene mutation (the F508del mutation). Cells contain two copies of the relevant gene, and Orkambi can be used in patients where both copies are affected by the F508del mutation. This equates to approximately 50% of people with cystic fibrosis, and according to the Cystic Fibrosis Trust, Orkambi is effective in treating 40% of these cases in the UK.

Orkambi has been shown to slow the decline in lung function and significantly reduce infection and hospital stays. The Cystic Fibrosis Trust highlights research which demonstrates that Orkambi can slow down the decline in lung function experienced by people with cystic fibrosis by an average of 42%.

The 2014 UK Cystic Fibrosis Registry report states that there are 2,834 people in England, 243 people in Scotland, 118 people in Wales and 101 people in Northern Ireland who could stand to benefit from Orkambi, a total of 3,296 people.

Medicines undergo an appraisal process to determine whether the benefit to patients justifies the cost, before they can be routinely used to treat NHS patients. The National Institute for Health and Care Excellence (NICE) advises the NHS on both the clinical and cost effectiveness of some newly-licensed medicines. This advice has a statutory basis in England and Wales, with Welsh health boards legally obliged to fund NICE-approved medicines. The All Wales Medicines Strategy Group (AWMSG) has a remit to appraise new medicines that are not on the NICE work programme. Health boards in Wales also have a legal requirement to fund medicines approved by AWMSG.

As noted in the petition, NICE guidance (July 2016) does not recommend the use of Orkambi within the NHS in England/Wales on the grounds of cost-effectiveness and a lack of long-term data. Orkambi is also not recommended (by the Scottish Medicines Consortium) for use in NHS Scotland. Due to NICE's negative recommendation, Orkambi is not available for routine commissioning in NHS Wales.

The NICE website states that the key conclusions on this drug were:

> Lumacaftor-ivacaftor is not recommended, within its marketing authorisation, for treating cystic fibrosis in people 12 years and older who are homozygous for the F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.
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> The committee concluded that longitudinal changes rather than acute changes in ppFEV1 were more clinically relevant for assessing long-term outcomes of cystic fibrosis. It also concluded that the reductions in pulmonary exacerbations seen with lumacaftor-ivacaftor treatment were clinically significant and important for managing cystic fibrosis.

> The committee concluded that, even without including any of its preferred assumptions, the estimated incremental cost-effectiveness ratios (ICERs) were considerably higher than what is normally considered a cost-effective use of NHS resources.

The next NICE review on Orkambi is due in **July 2019**. Medicines in Wales can also be appraised by the All-Wales Medicines Strategy group (AWMSG). However the AWMSG would require information in addition to that received by NICE, following a negative decision by NICE.

The Cystic Fibrosis Trust has been publicising the petition with case studies on its website, and is running campaign, *'Stopping the clock'*, on access to precision medicines like Orkambi. As described in the petition, the charity recently protested outside the Senedd about access to the drug.

## Response from the Welsh Government

The Cabinet Secretary for Health and Social Services states in his letter to the Committee that NICE issued final guidance in July 2016 and did not recommend Orkambi for routine use in the NHS in Wales or England. Furthermore **in December 2016, NICE re-issued its Technology Appraisal guidance under its "Do Not Do" guidance, emphasising that Orkambi should not be made routinely available.** The Cabinet Secretary highlights the significance of this:

> Whereas NICE frequently issue guidance which does not advocate using a specifics medicine for a certain condition, it is far rarer for NICE to explicitly advise that a medicine should not be routinely used at all. The NICE independent appraisal committee found that when compared to the current standard of care, **the clinical benefit offer** between NHS Wales, the AWMSG, WHSSC and Vertex Pharmaceuticals'; The Cabinet Secretary states that the AWMSG has contacted the pharmaceutical company, Vertex Pharmaceuticals, and 'has strongly encouraged them to make a submission to the AWMSG for appraisal'. He states:
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> Whilst Vertex has agreed in principle to submit clinical data for appraisal by AWMSG, they have not committed to any firm date for doing so. However, discussions have commenced with Vertex on the most effective approaches to appraisal for the additional license extensions due to come on stream over the next few years. My officials will ensure the future appraisal of lumacaftor/ivacaftor (Orkambi®) is covered.
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> In the interim, the Welsh Health Specialised Services Committee (WHSSC) has agreed a patient access scheme with Vertex Pharmaceuticals and it is available in the Welsh NHS, where clinically appropriate.
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> Whilst compassionate use agreements offer treatment at no cost for a fixed period, NHS organisations must consider the implications of entering into such agreements including the clinical benefits for patients and the longer term cost implication for the NHS.
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> Where medicines such as Orkambi are not routinely available within NHS Wales, a clinician may apply for the medicine on behalf of their patient to an Individual Patient Funding Request (IPFR) panel in the appropriate health board. The clinician would need to source sufficient evidence to demonstrate the clinical and cost effectiveness of the proposed intervention.