

## **P-05-797 Ensure access to the cystic fibrosis medicine, Orkambi, as a matter of urgency**

This petition was submitted by Rhian Barrance and was considered by the Committee for the first time in January 2018, having collected 5,717 signatures online.

### **Text of Petition**

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.

### **Additional Information**

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.

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

#### **Assembly Constituency and Region**

- Cardiff West
- South Wales Central