Llywodraeth Cymru
Welsh Government

WRITTEN STATEMENT
BY
THE WELSH GOVERNMENT

TITLE **Access to the cystic fibrosis medicines Orkambi® and Symkevi®**

DATE **12 August 2019**

BY **Vaughan Gething, Minister for Health and Social Services**

Access to medicines for patients with cystic fibrosis (CF) is a matter about which I and Assembly Members have regularly expressed deep concern. In February, I issued a written statement about access to the CF medicines manufactured by Vertex Pharmaceuticals. Today, in light of the announcement by the Scottish Medicines Consortium (SMC) that they cannot recommend the use of either Orkambi® or Symkevi®, I now wish to provide an update on the situation in Wales.

Cystic fibrosis is a serious inherited disease affecting around 400 people in Wales, mainly affecting the lungs and digestive system. It requires daily treatment including physiotherapy, antibiotics, and taking enzyme tablets with food, and it significantly reduces the quality and duration of people's lives. At present, there is no cure and current treatments aim to manage the individual symptoms.

CF has a devastating effect on patients and their families and obviously I share in their wish for access to effective new treatments for this chronic disease.

Let me be clear, my starting position is that I want to see new and innovative medicines made available promptly and consistently for patients in Wales. The £80m investment this Government is making in our New Treatment Fund is ensuring that is the case for all medicines appraised and recommended by the National Institute for Health and Care Excellence (NICE) and All Wales Medicines Strategy Group (AWMSG), two internationally recognised health technology assessment bodies.

Ivacaftor (Kalydeco®), the first disease-modifying treatment for patients with CF, has been available to patients in Wales since 2012. Since that time, its manufacturer Vertex Pharmaceuticals has brought two further CF medicines, lumacaftor/ivacaftor (Orkambi®) and tezacaftor/ivacaftor (Symkevi®) to market.

The availability of Orkambi® was considered by NICE and the SMC in 2016. Neither body was able to recommend its use then, and in 2017 NICE re-issued its recommendation as "Do Not Do" guidance, emphasising this treatment should not be routinely available.

Today's announcement by the SMC is clear that, despite new evidence and the agreement of a new discounted price, they remain unable to recommend the use of Orkambi®. Neither is SMC able to recommend the use of Symkevi®. In the case of both of these medicines, the SMC's decisions confirm the clinical and economic cases presented were insufficiently robust to allow either medicine to be considered cost-effective, even at the discounted price offered by Vertex.

Since 2016, we have repeatedly invited Vertex to engage in the AWMSG appraisal process. Eventually in June, Vertex wrote to me confirming their intention to make a submission to AWMSG in respect of both Orkambi® and Symkevi®. However, to date Vertex has still not sent AWMSG a submission for either medicine.

Our NHS relies on evidence-based appraisals to ensure that its limited resources are used to best effect. This latest decision by an expert appraisal body adds to the evidence that Vertex's pricing does not reflect the clinical effectiveness of their medicines, and that the NHS is right not to consider them as a routine treatment for CF. I urge Vertex to reflect on their pricing mechanism and the robustness of their clinical data, before making an urgent submission to AWMSG.

This statement is being issued during recess in order to keep members informed. Should members wish me to make a further statement or to answer questions on this when the Assembly returns I would be happy to do so.