# WRITTEN STATEMENT

# BY

# THE WELSH GOVERNMENT

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| **TITLE** | Access to the cystic fibrosis medicine Orkambi® (lumacaftor/ivacaftor) |
| **DATE** | **01 March 2019** |
| **BY** | Vaughan Gething, Minister for Health and Social Services |

Cystic fibrosis (CF) is a serious inherited condition where a gene defect causes a person’s lungs and digestive system to become clogged with sticky mucus. This leads to damaging blockages. Symptoms include a persistent cough, recurring infections, malnutrition and other serious complications such as diabetes. At present, there is no cure for cystic fibrosis and current treatments aim to manage the individual symptoms.

Naturally I share the concerns of patients and their families and their desire for access to effective new treatments for this debilitating disease.

The NHS uses an evidence-based approach to decide which treatments work best for patients, and are also cost-effective. There are long-established, robust processes to implement this approach. Firstly, if a pharmaceutical company wants to sell a medicine in the UK, they must have a licence from the Medicines and Healthcare products Regulatory Authority (MHRA). This is mandatory. The MHRA examines all the evidence the company has that the medicine is safe and effective, and if the evidence is strong enough, the MHRA grants a licence. Licensed medicines are available on the NHS in Wales.

Secondly, in the UK a licensed medicine may be appraised by any of several appraisal bodies namely the All-Wales Medicines Strategy Group (AWMSG), the National Institute for Health and Care Excellence (NICE) and the Scottish Medicines Consortium (SMC). This process is voluntary and entirely at the manufacturer’s discretion. Each of these independent expert bodies scrutinise the manufacturer’s evidence of the medicine’s clinical effectiveness in treating the medical condition, as well as its cost-effectiveness in comparison with existing treatments, taking into account input from clinicians, other healthcare professionals and patients. If AWMSG or NICE recommend the medicine, it becomes routinely available on the NHS in Wales. If they do not, the medicine is not a standard treatment, but can still be provided via an Individual Patient Funding Request (IPFR).

Vertex Pharmaceuticals manufacture several medicines to treat cystic fibrosis, including Orkambi® (lumacaftor/ivacaftor). They chose to have Orkambi®, for patients aged 12 and over, appraised by NICE. In July 2016, NICE did not recommend it. NICE concluded the Orkambi® was not cost-effective. The SMC in Scotland also appraised Orkambi® in 2016 but did not recommend it. In 2017, NICE re-issued its recommendation as “Do Not Do” guidance, emphasising this treatment should not be routinely available.

In November 2017 the Petitions Committee received a petition about Orkambi® and was told that Vertex had new evidence of its clinical effectiveness. Instead of presenting the new evidence to NICE, who carried out the original appraisal, Vertex said that they would send it to AWMSG. I wrote to Vertex last year urging them to fulfil this commitment. To date no new evidence has been submitted to AWMSG.

In October 2018 Vertex asked NICE to appraise Orkambi® for treating patients aged 2-11. In December 2018 Vertex asked the SMC to appraise Orkambi® for the same group of patients. In January 2019, Vertex stated that NICE’s well-established appraisal process – used to successfully appraise medicines for two decades – was not appropriate for their medicines.

Without the evidence-based approach - a clear set of criteria and independent clinical experts to appraise the clinical and cost-effectiveness of new medicines - the NHS would have no way of identifying medicines which are the most cost-effective and most clinically effective, or even distinguishing between them. The evidence-based approach that is in place is the safest method for ensuring the most effective treatments for patients and the most effective use of NHS resources. I encourage pharmaceutical companies to engage in the appraisal process and I would ask Members to support this approach.