

Cystic Fibrosis Drugs: Orkambi

Sarah Wollaston Chair, Health and Social Care Committee, Chair, Liaison Committee (Commons), Chair, Liaison Committee

I thank my constituent, Cathy Meredith, who started this petition. I also thank Oli Rayner and the many others living with cystic fibrosis who, sadly, cannot be in the Public Gallery with us today because cystic fibrosis is such a curiously isolating condition—those suffering from it cannot be in the same room as others because of the risk of transmitting resistant infections.

I will start with some context on the cause of cystic fibrosis, which is a mutation in the cystic fibrosis transmembrane conductance regulator gene, affecting the production of a protein that in turn has consequences for the balance of salts and fluids moving across membranes, leading to an accumulation of thick, sticky mucus in the lungs and other organs. The point, however, is that although 10,000 people in the UK live with cystic fibrosis, it is not really a single condition: there are many mutations of the CFTR gene. That has consequences for the types of medication to which people will best respond. We need to think of cystic fibrosis not only as a rare genetic condition but as a series of much rarer conditions. That is important to note.

We now have some real hope for progress with the CFTR modulators, but we need to make that progress much more rapidly than we are. The negotiations between Vertex and NHS England have dragged on for far too long. The patients living with cystic fibrosis and their families, have been lost in those discussions. We need not only to return to thinking about them, but to bear in mind the implications that go far beyond those living with cystic fibrosis.

The NHS has a responsibility to consider the wider cost of drugs, including the opportunity costs—what we cannot treat if our NHS budget is consumed completely by the ever-rising cost of drugs. For the NHS to have that responsibility is a tough message for all of us, which is why we need bodies such as NICE to make the decisions to ensure fairness for all patients who rely on NHS resources. To put that in context, the drugs budget in 2017-18 was £18.2 billion. A little more than half of that was for hospital drugs and, over the past seven years, the costs of those drugs have increased by 119%. We therefore have to bear in mind the implications of taking a free-for-all approach to drugs costs, which the Minister will not want to do.

The Government are trying to get the parties around the table. Unfortunately, the gap is huge between what Vertex continues to demand for the drugs and what the NHS is offering based on recommendations from NICE. The gap is not small; it is considerable. Other companies have come to the table to negotiate their prices, so I call on Vertex to look again at what is happening. It is absolutely disgraceful that families have to resort to such things as buyers' clubs; the inequalities that that creates are horrific. We need Vertex to focus on what is happening.

I am also concerned about some of the points made during our Health and Social Care Committee inquiry into Vertex. For example, we asked the company directly whether drug

supplies had been destroyed because they were going off date, and we were told that that was not the case and was very unlikely to happenâ€”but it has been happening. That is wholly unacceptable.

To come back to the alternatives, Kerry McCarthy touched on the issue of Crown use licences, for example. One of the areas that our Committee considered was possible referral to the Competition and Markets Authority. In fact, we have now heard that that would take many years, so unfortunately the area does not look like one we can pursue further. However, given so little progress since our inquiry, the Committee wrote to all the parties involved in the negotiationsâ€”NHS England, Vertex and NICEâ€”to ask where we are now.

The most promising idea that we should take forward to apply pressure is that of interim agreements, such as in Scotland. An interim price is agreed, further research is carried out and all parties agree to a review based on the outcomes of that further research. That is being managed in Scotland and other places, as we have heard from other speakers today, and I urge Vertex to do that here. We all recognise the need for a fair price to enable further research to take place. We all recognise that many other drugs are in the pipeline, particularly a very promising triple therapy, which NHS England has now agreed to take off the table so that it does not distort future pricing. That is a sensible thing to do at this stage, so that within the current offer we look just at the three existing treatments. At a later stage, we can come back to look at the triple therapy evidence. I urge all parties to come to an interim agreement at least, and to continue to put patients front and centre in everything they do.

Finally, I would like to touch on the political aspect, because both President Trump and US Secretary of State Azar have repeatedly referred to using their muscle in trade negotiations to increase the price that European countries would have to pay for their drugs. They have referred to the NHS "freeloading", for example. That is very worrying. We all need to be aware of the dangers of a future trade deal and the implications that it could have on negotiations for a range of other products. I hope that those points have contributed to the debate. All parties need to focus on the people at the heart of the issue: the patients who are living with cystic fibrosis.

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The Minister referred to the importance of Vertex engaging with NICE. Does she share my concern that when I wrote to Vertex and NICE about the failure to make progress, Vertex assured me that it had contributed "substantial new evidence" on the three products in question, yet I subsequently heard from NICE that it had received only

"an overview of the clinical evidence", rather than genuine engagement? Will she join me in calling on Vertex to properly engage with the process, so that we can get the full evidence base on which to make these decisions?