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## News

### Landmark NHS deal to open up access to life-changing cystic fibrosis drug

21 August 2020

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**From today thousands of NHS cystic fibrosis patients in England can benefit from a 'transformative' treatment for cystic fibrosis.**

NHS patients will be among the first in Europe to be prescribed Kaftrio, which significantly improves lung function, helping people with cystic fibrosis to breathe more easily and enhancing their overall quality of life.

The treatment – known as the 'triple combination therapy' – was given the green light by European regulators today, setting live a deal struck by NHS England to get the drug onto the frontline of patient care as soon as it was licensed.

NHS chief executive Simon Stevens announced the agreement at the Health Select Committee on the 30 June this year.

The [UK Cystic Fibrosis Medical Association](#) has described the treatment as showing evidence of being potentially 'truly life-transforming'.

The immediate availability of Kaftrio, alongside existing treatment options previously negotiated between NHS England and Vertex Pharmaceuticals, means that most patients with cystic fibrosis – more than 7,000 people in England – can benefit from a therapy which tackles the underlying causes of the condition.

As part of the innovative commercial deal, NHS England also secured equivalent terms for cystic fibrosis patients in Wales, Northern Ireland and Scotland paving the way for full UK access.

Welcoming the news that the NHS can start prescribing the new treatment, **NHS national medical director Professor Stephen Powis**, said: "At the same time as staff came together to put in a herculean effort to treat over 108,000 for coronavirus in hospitals, they also continued providing round the clock care for patients with long term conditions such as cystic fibrosis, and this landmark deal has put NHS patients at the front of the queue for transformational treatment.

"The NHS has developed world-leading commercial capabilities, enabling us to strike such landmark deals with the pharmaceutical industry and support people with cystic fibrosis to lead longer, healthier, better quality lives."

**David Ramsden, Chief Executive of the Cystic Fibrosis Trust**, said: "The licensing of Kaftrio today marks a step change in the treatment of cystic fibrosis. With agreements between Vertex and governments across the UK already in place, thousands of eligible people across the UK can now discuss with their clinical team how they can start this treatment as soon as possible.

"It's also great news that more mutations have been added to those eligible for Kalydeco and Symkevi, giving more children and adults with CF a disease modifying treatment option.

"The UK CF Registry will continue to support access to and monitoring of these new therapies for people with cystic fibrosis. It's thanks to a huge community effort that these medicines are now available for so many people with cystic fibrosis in the UK, and as quickly as possible.

"At such a positive moment, we are also mindful that much work still remains to find drugs appropriate for all people with cystic fibrosis and further breakthroughs that will support everyone to truly live a life unlimited."

The terms of the deal landed by NHS England also mean that as many as 300 patients with some rarer genetic mutations, which fall outside of the scope of the European licensing body's considerations, will now be able to benefit from Kalydeco and Symkevi, also produced by Vertex Pharmaceuticals, offering a lifeline to those with limited treatment options.

Today's watershed moment builds on previous NHS support given to people with cystic fibrosis that saw thousands of people with the condition receive devices allowing medics to monitor their condition remotely, as part of the NHS drive to give more people connected, supported and personalised care in their own homes, saving them from having to make trips to hospital.

The deal for the triple combination therapy was made possible by the NHS working in partnership with the [National Institute for Health and Care Excellence \(NICE\)](#), the internationally renowned body for assessing the clinical and cost effectiveness of new treatments and technologies.

The deal will last for four years to allow for further data to be collected to better understand the impact for patients of this game-changing treatment.

In clinical trials Kaftrio has been shown to significantly improve the lung function of cystic fibrosis patients with two copies of the F508del mutation or one copy of the F508del mutation with a Minimal Function mutation, enabling them to breathe more freely and enhancing their overall quality of life.

NICE will continue to work with the company and the NHS on ongoing data collection for Kaftrio, Orkambi, Symkevi and Kalydeco. Prices will then be adjusted as necessary, following full NICE appraisal, to ensure that taxpayers continue to get a good deal.

As the European licence for the triple combination will only cover patients 12 years and older at this stage, younger children and those over 12 years who are not eligible for Kaftrio will continue to benefit from access to other cystic fibrosis drugs where clinically appropriate, with access to the triple therapy to follow if regulators approve a broader license in the future.

Investment in world-class, cutting edge technologies and treatments is at the heart of the NHS Long Term Plan, published last year.

The deal for the Kaftrio is the latest in a growing catalogue of commercial access deals benefiting thousands of patients that have been put in place in recent years, including new drugs to eliminate Hepatitis-C, CAR-T therapy to treat children and adults with certain cancers and a treatment for children with sight loss, onto the front line of NHS care in England.

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