

Availability of new treatments for cystic fibrosis in the UK

The UK Cystic Fibrosis Medical Association (UKCFMA) are very pleased to hear that NHSE have signed a commercial agreement with Vertex for the triple therapy, known as Kaftrio, the day EMA licensing is approved. We are excited that our patients will be able to benefit from this remarkable treatment and would like to thank NHSE, Vertex and everyone else involved for coming to a swift agreement to make this drug available. This agreement includes the flexibility for clinicians to use drugs in the portfolio for rare mutations, as approved by the FDA, which means clinicians will be able to ensure that as many eligible people with cystic fibrosis (CF) as possible can benefit from this treatment in England.

We understand that this agreement also includes 'tag along' rights for Scotland, Wales and Northern Ireland and would urge the relevant representatives of those governments to put an access agreement in place across the devolved nations as soon as possible. We are monitoring the situation and inputting where appropriate to ensure no one is left behind.

It is difficult knowing that not everyone will be eligible for Kaftrio or the other licensed modulators. Research and clinical trials are still ongoing for the remaining 10% of the population to ensure a treatment is found as soon as possible.

If you have any questions about Kaftrio or aren't eligible for a modulator but want to know more about clinical trial opportunities, please contact your CF team.