UK Cystic Fibrosis Medical Association
UKCFMA

UK CF Medical Association statement on the triple therapy delay – 10 June 2020

Triple CFTR modulator therapy ("Trikafta"), a treatment for the basic defect in cystic fibrosis (CF), is currently under review by the European Medicines Agency (EMA) and in the UK by the National Institute of Clinical Excellence (NICE). The CF Medical Association (CFMA), amongst others, has contributed to the NICE review, recognising the importance of making this therapy available to people with CF at the earliest possible opportunity.

The NICE review was originally planned to be completed by the end of 2020. Along with many in the CF community, CFMA were surprised and disappointed to hear recently that this process has been delayed to allow submission of more clinical data in support of the review. CFMA understands that, in parallel to the NICE review, Vertex are in negotiation with the Department of Health.

The EMA now have all the data they require for their decision-making process and approval is expected before the end of this year. This would grant Trikafta a licence in the EU and UK. The current coronavirus pandemic, the vulnerability of people with CF and the implications of shielding have brought the urgent need for more effective therapies into sharp focus. Trikafta leads to improved lung function and fewer hospital admissions and is thus one of the few effective therapies that may help patients safely reduce shielding requirements and aid return to school and work.

On behalf of the CF medical community, the CFMA urge all parties to continue constructive discussions to achieve an early resolution across all the UK nations and allow access to this drug as soon as possible.