

UK Cystic Fibrosis Medical Association

UKCFMA

Rt Hon Matt Hancock MP
Secretary of State for Health and Social Care
Department of Health and Social Care
39 Victoria Street
London
SW1H 0EU

30th January 2020

Dear Mr Hancock

Re: Availability of new treatments for Cystic Fibrosis in the UK

The executive committee of the UK Cystic Fibrosis Medical Association (UKCFMA) would like to thank-you, on behalf of all consultants treating people with CF, for your efforts to secure a definitive agreement with Vertex Pharmaceuticals to make available all three of their UK-licensed CF medicines.

The UKCFMA represents more than 200 CF doctors working in paediatric and adult respiratory medicine throughout the UK. As a result of the agreement reached in October last year, you rightly stated that we are now able to improve the lives of thousands of our patients through the availability of these medicines. This is a hugely exciting time, with the genuine prospect of ever improving treatments that correct the underlying defects in the cells of our patients.

We were particularly pleased to learn that in reaching agreement with Vertex, NICE successfully secured a binding condition that the company would submit for comprehensive appraisal its new medicine, the triple combination therapy (known as Trikafta in the US), at the earliest opportunity. This is the most recent of Vertex's CF medicines and was approved for use by the US Food and Drug Administration in October of last year.

Whilst the recent availability of these new medicines will result in significant improvements for our patients, the real life experiences of people with CF receiving the latest triple combination drug in America, suggest it is truly life-transforming. Those of us who have been fortunate in having patients participate in the clinical trials for its development, have seen a beneficial effect beyond the magnitude that we might ever have anticipated. Many of our patients no longer feel as though they have CF after receiving this medicine. Furthermore, Trikafta has literally been life saving when obtained through Vertex's managed access programme on a compassionate basis to treat patients with end stage disease.

We urge you to continue making all practical efforts within your department to ensure that Trikafta is made available at the earliest opportunity. In particular, we would ask that you ensure that Vertex honour their commitment to positively engage with NICE and its processes to secure early access to Trikafta at a fair price, as has been achieved for many other new treatments.

The UK has the second highest incidence of CF in the world and we are proud of the excellence of our CF multi-professional NHS teams which we believe are the best in the world. We hope that we can continue to be at the forefront of clinical medicine and provide our patients with the very best available treatments.

Thank-you for your consideration of these matters. We would value the opportunity to meet and discuss the best course of action towards accessing this new triple combination therapy for our patients.

Yours sincerely,



Dr Caroline Elston
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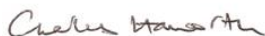
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