Statement from the UKCFMA to help people understand the current situation with regard to the availability of CFTR modulator treatment in the UK

11 February 2021

NHS England has this week provided updated information for CF clinicians about the prescribing of CFTR modulators.

They have done this to move towards a more level playing field for access to CFTR modulators across the UK and to update clinicians about developments to meet the needs of patients who might derive benefits from the best available CFTR modulators but whose genotype is not included within current UK licensing.

The updated information reassures physicians that if they decide it is necessary to prescribe a CFTR modulator (i.e. Kaftrio) to meet the needs of a patient with one copy of a DF508 mutation but for whom this drug is not currently licensed, they will be acting within General Medical Council Guidance for best practice. The costs of the drug will be reimbursed. This includes patients not doing well despite treatment with other CFTR modulators (i.e. Ivacaftor and Symkevi).

Whilst these arrangements are in place, NHS England is working towards provision for all CF patients who might benefit from CFTR modulators in UK. They have informed us that they are awaiting decisions by the European Medicines Agency (EMA) about whether it will extend the licence of Kaftrio for patients with the DF508 mutation plus any other mutation. Vertex has submitted clinical data to support the extension of this licence which the EMA are considering. If this submission is successful, it will usefully formalise the above prescribing arrangements to within the product's marketing authorisation.

Unfortunately, the EMA will not take into consideration the *in vitro* data that Vertex submitted to the FDA (the American licensing body) pertaining to patients without a DF508 mutation. Nevertheless, NHS England is reviewing this evidence independently of the EMA and will be deciding as soon as possible whether to extend UK commissioning of Kaftrio for patients with specific gene mutations other than DF508.

People with CF aged over 12 years should discuss the implications of these ongoing developments with their CF centre.