Resource impact statement

Resource impact
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NICE has recommended:

- ivacaftor–tezacaftor–elexacaftor (IVA–TEZ–ELX) plus ivacaftor (IVA) alone within its marketing authorisation, as an option for treating cystic fibrosis (CF) in people 2 years and over who have at least 1 F508del mutation in the CF transmembrane conductance regulator (CFTR) gene.
- tezacaftor–ivacaftor (TEZ–IVA) plus IVA alone, within its marketing authorisation, for treating CF in people 6 years and over who have:
  - 2 copies of the CFTR gene with F508del mutations or
  - a copy of the CFTR gene with an F508del mutation and a copy of the CFTR gene with 1 of the mutations listed in section 2.2 of the guidance.

- lumacaftor–ivacaftor (LUM–IVA), within its marketing authorisation, for treating CF in people 1 year and over who have 2 copies of the CFTR gene with F508del mutations.

The treatments are already in use in the NHS. In 2019, an agreement was reached between NHS England and Vertex to make LUM–IVA and TEZ–IVA available on the NHS while more evidence was collected. In 2020, the agreement was updated to include IVA–TEZ–ELX. Based on 2021 data from the UK Cystic Fibrosis Registry, 72.6% of people with CF were taking a CFTR modulator and, of those, 72.1% were taking IVA–TEZ–ELX. The proportion taking IVA–TEZ–ELX had increased to 86% by December 2022. We now estimate this to have increased to 93% in the eligible population.

Potential resource benefits already realised while the treatments have been in use include:

- fewer medical interventions and less time in hospital for people receiving the treatments. Around 38% of people had hospital treatment for pulmonary exacerbations in 2019 (Cystic Fibrosis Trust. UK Cystic Fibrosis Registry: 2019 Annual Data Report. 2020) versus around 1.5% for people taking the treatments based on trial data.
- reductions in lung transplants for this population group. Although not a capacity impact, other people on the transplant waiting list would benefit. Table 1 uses data provided from NHS Blood and Transplant for the reduction in lung transplants in people with CF after the introduction of CF transmembrane conductance regulator modulators (CFTRms).

Table 1 Average number of lung transplants per year before and after the introduction of CFTRms

–	Before CFTRms (period 1 April 2013 to 31 March 2020)	After CFTRms (period 1 April 2020 to 31 March 2023)
Adults	47 (26.1%)	8 (7.8%)*
Children	4 (51%)	0

The percentages are the proportion of CF-related lung transpants from the total number of lung transplants for any reason over each period.

The company has a commercial arrangement. This makes the technologies available to the NHS with a discount. The size of the discounts are commercial in confidence.

A local template is provided for services to assess the resource impact locally.

The technologies in this appraisal for people with CF are commissioned by NHS England. Providers are NHS hospital trusts.

The payment mechanism for the technologies are determined by the responsible commissioner and depends on the technologies being classified as high cost.