Cardiothoracic Transplant Patient Group at NHSBT

NICE Consultation Response

Ivacaftor–tezacaftor–elexacaftor, tezacaftor–ivacaftor and lumacaftor–ivacaftor for treating cystic fibrosis [ID3834]

The Cardiothoracic Transplant Patient Group (CTPG) strongly supports continued access to CFTR Modulator Therapies for all clinically appropriate people with Cystic Fibrosis (CF).

The CTPG represents all patients in the UK at any stage of heart and / or lung transplant pathways. As such the CTPG will focus their response on the impact on the whole lung transplant patient community.

Lung transplantation offers a live saving treatment option to some patients with end stage lung failure. Patients are referred to lung transplantation from several different disease types.

Historically cystic fibrosis patients have represented a sizeable proportion of patients referred for lung transplant. The introduction of CFTR Modulator Therapies for Cystic Fibrosis has significantly reduced the demand for lung transplants from this patient group.

In the 7 full years from 1 April 2013 to 31 March 2020 on average 65.7 adults with CF were added to the lung transplant waiting list each year, this represented 22.2% of the total patients during that period. Since the introduction of triple therapy in 2020, the demand for lung transplantation from CF patients has dropped. (Data from NHSBT)

In the following three full years from 1 April 2020 to 31 March 2023, an average of 9 adult patients with CF have been added to the lung transplant waiting list each year, this represents 5.2% of total patients added to the waiting list during that period. (Data from NHSBT)

A similar impact has been seen in paediatric lung transplant waiting list registration.

In the 7 full years from 1 April 2013 to 31 March 2020 on average 2.86 children with CF were added to the lung transplant waiting list each year, this represented 38.5% of the total patients during that period. Since the introduction of triple therapy in 2020, the demand for paediatric lung transplantation from CF patients has dropped. (Data from NHSBT)

In the following three full years from 1 April 2020 to 31 March 2023, an average of 0.3 children with CF have been added to the lung transplant waiting list each year, this represents 4.2% of total patients added to the waiting list during that period. (Data from NHSBT)

In terms of lung transplants undertaken, a lag in the change would expect to be witnessed, however, the actual change has been equally stark.

In the 7 full years from 1 April 2013 to 31 March 2020 on average 47 adults with CF underwent lung transplantation each year, this represented 26.1% of the total patients transplanted during that period. Since the introduction of triple therapy in 2020, the demand for lung transplantation from CF patients has dropped. (Data from NHSBT)

In the following three full years from 1 April 2020 to 31 March 2023, an average of 7.7 adult patients with CF have undergone lung transplantation each year, this represents 7.8% of total patients transplanted during that period. (Data from NHSBT)

In the 7 full years from 1 April 2013 to 31 March 2020 on average 3.7 children with CF underwent lung transplant each year, this represented 51.0% of the total children transplanted during that period. Since the introduction of triple therapy in 2020, the demand for paediatric lung transplantation from CF patients has dropped. (Data from NHSBT)

In the following three full years from 1 April 2020 to 31 March 2023, no children with CF have undergone lung transplantation. (Data from NHSBT)

The CTPG anticipate the decreased requirement for lung transplantation from CF patients will continue if access to CFTR Modulator Therapies for all clinically appropriate people is maintained.

If, as is recommended by NICE, access to CFTR Modulator Therapies for new patients is denied then demand for lung transplantation from CF patients will inevitably rise.

For almost all medical therapies capacity can be, at least in theory, increased to meet demand. The same is not true for solid organ transplantation, as it entirely relies on organ donation.

Despite current and continuing efforts to increase organ donation and utilisation, supply will never meet demand and many patients will die whilst waiting for organ transplantation.

This is especially true in lung transplantation. For adult patients added to the routine lung transplant waiting list between 1 April 2018 and 31 March 2020, at 3 years after registration 36% had died or been removed from the waiting list (predominately due to deterioration), compared to 39% who had received a transplant. At two out of the five adult lung transplant centres, routine patients at one year after registration were more likely to have died or been removed from the waiting list than received a transplant. (Data from NHSBT)

Any therapies that can reduce the need for lung transplantation must be considered in a wider context than the patients they directly impact on.

Since the introduction of CFTR Modulator Therapies for CF patients, the overwhelming majority of donated lungs have been allocated to patients with other disease presentations, the most common being pulmonary fibrosis. This is affording significant lung transplant access benefits to non-CF disease types that do not have treatment options as effect as the CFTR Modulator Therapies for CF patients.

Whilst NICE have acknowledged the knock-on impact of CFTR Modulator Therapies for CF patients on lung transplantation they have not qualified this nor, and most importantly considered the impact on other disease types which present for lung transplantation.

The CTPG believe NICE have failed to consider all the relevant evidence as they have not calculated the impact on lung transplant services and the significant tangible benefits CFTR Modulator Therapies for CF patients provide to patients with other lung disease types presenting for transplantation, such as pulmonary fibrosis.

The CTPG believe NICE have failed to undertake an appropriate cost effectiveness assessment of CFTR Modulator Therapies for CF patients as they have not quantified or financially assessed the benefits afforded to other patient groups who will derive significantly better access to lung transplantation. The CTPG believes this failure has significantly overestimated the ICER NICE have calculated in the appraisal.

The CTPG believe NICE have demonstrated a fundament lack of understanding in the underlying UK demand for donated lungs by stating that CFTR Modulator Therapies for CF

patients reduce the need for “transplanted organs themselves”. This is not the case as demand significantly outstrips supply.

The CTPG appreciate that the calculation of indirect benefits on the health service and other patient groups is often challenging to appropriately quantify and hence include in cost calculations.

However, lung transplantation is an almost unique scenario in that demand for organs significantly outweighs an externally limited supply. This scenario is likely to continue for at least the medium term (decades).

This scenario effectively means that for every CF patient not requiring lung transplantation the organs can be allocated to a non-CF patient who will derive the full life extending benefit with no cost attributed (as their lung transplant treatment and follow up costs would have been incurred by the transplanted CF patient, except for any case mix adjustment).

The CTPG believe NICE must calculate and include this impact in their financial assessment to ensure a more appropriate and accurate ICER is derived for the appraisal of CFTR Modulator Therapies.