

CFNZ TRIKAFTA KEY MESSAGES – updated 8 February 2022

Current Situation

- Vertex Pharmaceuticals (Vertex) applied to Pharmac for funding of Trikafta in New Zealand in July 2021.
- Pharmac’s Respiratory Subcommittee considered Trikafta and recommended it as a high priority for funding.
- The recommendation is for people with CF who are 6 years or older who have one or more copy of the F508 del mutation or other mutation responsive to Trikafta.
- Following this recommendation and an economic health assessment, Pharmac announced in October 2021 that it had added Trikafta to the list of medicines that it wants to fund – its Options for Investment list¹.
- Pharmac described Trikafta as a “paradigm-shifting” treatment for cystic fibrosis (CF).
- Pharmac’s Pharmaceutical and Therapeutics Committee (PTAC) considered Trikafta in November 2021 to provide additional advice on how it should be ranked against other medicines Pharmac wants to fund.
- CFNZ expects the outcomes from Pharmac’s November 2021 PTAC meeting to be released in February 2022.

CFNZ Position

- Pharmac’s assessment of Trikafta clearly demonstrates it would bring life-changing benefits to Kiwis with CF by substantially improving their quality of life and life expectancy and enabling them to live almost normal lives.
- Trikafta would also deliver major benefits to the NZ health system, through significant reductions in medical treatment, hospitalisations, and lung transplants.
- Real world data recently published by Vertex covering more than 16,000 CF patients in the US reinforces Trikafta’s effectiveness, and the life-changing benefits it could bring to Kiwis with CF ²:
 - 84% reduction in the risk of lung transplant

¹ Pharmac, 27 October 2021

² Vertex , January 2022

- 77% fewer pulmonary exacerbations (serious lung infections)
- 74% reduction in the risk of death
- no reduction in lung function after 96 weeks (almost 2 years) on Trikafta.
- The high priority recommendation from Pharmac’s clinical advisors, the positive economic assessment and the compelling evidence of benefit, mean that Trikafta needs to be funded as soon as possible.
- CFNZ urges Pharmac to engage with Vertex to agree a fair and reasonable price for Trikafta
- CFNZ urges Government to make funding available to Pharmac to ensure everyone in New Zealand who would benefit has access to Trikafta. Kiwis with CF deserve a future to look forward to.

Background Information

Cystic Fibrosis

- Cystic fibrosis (CF) is a serious and ultimately terminal genetic condition which affects more than 540 people in NZ.
- CF causes the body to produce thick, sticky mucus that results in serious clinical consequences for multiple organs, including the lung, pancreas, liver, intestines, and the reproductive system.
- People with CF experience frequent pulmonary infections, malnutrition, and other complications such as CF-related diabetes that substantially reduce their quality of life and life expectancy.
- Current treatment in New Zealand focuses on the symptoms of CF, with the aim of slowing the progression of the condition and enhancing quality of life.
- People with CF endure a life-long, demanding and time-consuming daily regime of lung clearance and medication, together with regular medical check-ups, increasingly frequent hospitalisations, and in some cases lung transplantation. The daily treatment burden for CF is high – up to 3 to 4 hours per day - and increases with age and severity of the condition.
- The lives of those with CF are cut short by around 50 years. Only half of those with CF reach 31 years of age, compared with 80 for the New Zealand population as a whole.
- CF has a major negative impact on the emotional and mental well-being of those with CF, their families and whānau, due to the stress, anxiety, fear and grief of living with a serious illness and facing early death. This is compounded for the 50+ families in New Zealand with more than one person with CF.

Trikafta

- Trikafta (elexacaftor/tezacaftor/ivacaftor) is manufactured by Vertex Pharmaceuticals.
- Trikafta treats the underlying cause of CF and works for around 90% of those with CF.
- Trikafta is already now available to CF patients in 22 countries, including the UK, Republic of Ireland, the USA, Denmark, Germany and Switzerland. It is currently being considered for funding in Australia.

- Medsafe gave consent for the distribution and use of Trikafta in New Zealand in December 2021. Medsafe gave the application priority status and consent was granted within 6 months.

Benefits of Trikafta for People with CF

- Trikafta provides significant, immediate, and enduring improvements for those with CF by increasing lung function and weight gain, reducing the burden of treatment, improving quality of life, and increasing life expectancy.
- Pharmac's respiratory experts concluded that Trikafta is a paradigm-shifting treatment for CF and that it:
 - Provides patients with substantial improvements in quality of life and an expectation of substantial prolongation of life
 - Could allow patients with CF to live a nearly normal life.
- The experts also concluded that Trikafta could provide substantial and meaningful improvements in quality of life and symptom burden for both those with progressed disease and younger patients who have not yet developed significant organ damage or lung impairment.
- A number of New Zealanders have been able to access Trikafta by self-funding or through Vertex's Managed Access Programme. As a result, they have experienced life-changing improvements in their health, well-being and ability to fully participate in life.

Benefits to the NZ Health System

Pharmac's respiratory experts concluded that Trikafta could:

- Reduce lung transplants for CF by 80% over time
- Reduce hospitalisations for pulmonary infections
- Reduce current treatments by 80% over time
- Substantially reduce antibiotic use, particularly for intravenous and nebulised antibiotics
- Reduce the need for dornase alfa, hypertonic saline and chest physiotherapy.