

## **TRIKAFTA KEY MESSAGES**

### **CFNZ's Key Messages**

- CFNZ welcomes Pharmac's announcement that it wants to fund Trikafta.
- CFNZ appreciates how quickly Pharmac has progressed Vertex's application for Trikafta.
- CFNZ acknowledges the difficult decisions Pharmac has to make when deciding which medicines to fund from its limited budget.
- CFNZ shares Pharmac's view that Trikafta is a paradigm-shifting treatment for cystic fibrosis (CF).
- Investing in Trikafta would bring life-changing benefits to nearly 400 Kiwis with CF, their families and whānau by substantially improving their quality of life and life expectancy and letting them live an almost normal life.
- Investing in Trikafta would also deliver substantial benefits to the NZ health system, through significant reductions in medical treatment, hospitalisations, and lung transplants.
- CFNZ encourages Pharmac to fully recognise the life-long burden faced by Kiwis with CF, and how Trikafta would lift that burden, by ranking Trikafta very highly on its Options for Investment list.
- CFNZ also urges Pharmac and Vertex to progress their commercial negotiations as quickly as possible to agree a fair and reasonable price for Trikafta – and make sure that Kiwis with CF have 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.

### *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.

### *Pharmac and Trikafta*

- Pharmac's respiratory experts have reviewed the evidence for Trikafta and have recommended that it have a high priority for funding. The recommendation is for those people with CF who are 6 years or older and who have a CF mutation responsive to Trikafta.

- Pharmac has carried out an economic assessment of Trikafta and decided to add it to the list of medicines it would like to fund – its Options for Investment list.
- Pharmac has described Trikafta as a “paradigm-shifting” treatment for CF as it treats the cause of CF rather than the symptoms.
- Pharmac’s Pharmaceutical and Therapeutics Advisory Committee (PTAC) will consider Trikafta at its meeting on 18/19 November 2021 and provide advice to help determine how Trikafta ranks against the other medicines Pharmac wants to fund.
- Before it can be funded, Pharmac needs to negotiate an agreement with Vertex, consult on its proposal to fund, and ensure it has sufficient budget.