SUBMISSION

TO THE HEALTH SELECT COMMITTEE

DUE MONDAY 2 MAY 2022

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Submission to the Health Select Committee in support of the petition from Carmen Shanks.

This submission is in support of a petition circulated and signed by 43,410 signatures. This petition asked:

That the House of Representatives urge the Government to publicly fund Trikafta for people in New Zealand with cystic fibrosis.

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INTRODUCTION

We thank the Health Select Committee for the opportunity to provide a supporting submission.

I write on behalf of Cystic Fibrosis NZ to support the petition of Carmen Shanks to have Trikafta medication publicly funded for people in New Zealand with cystic fibrosis (CF).

Cystic fibrosis (CF) is an inherited genetic condition that affects more than 540 people in New Zealand. You are born with CF – you cannot catch it. CF imposes a life-long, demanding regime of medical treatment, hospitalisations and, for some, lung transplant, and premature death. Current treatment for most people with CF in New Zealand deals with only the symptoms of CF, not its cause, and existing treatments can only slow the decline in health experienced by the majority of those with the condition.

Manufactured by US biotech company, Vertex Pharmaceuticals, Trikafta is the first medicine to treat the cause of CF for the majority of those with the condition. Trikafta would provide substantial improvements in quality of life and an expectation of substantial prolongation of life for the majority of people with CF in New Zealand, enabling them to live almost normal lives.

Our submission describes the substantial challenges faced by those living with CF in New Zealand under current treatment regimes, and the substantial benefits that Trikafta would bring. Our submission also addresses the need for improvements to medicines' funding arrangements in New Zealand. Public funding of Trikafta would fundamentally change the lives of the majority of those with CF in New Zealand, their families and whānau.

People with CF don’t have time to wait.

CYSTIC FIBROSIS NZ

Cystic Fibrosis NZ is a charity dedicated to supporting the CF community by providing information, advice, and financial assistance to those with CF and their families throughout their journey with CF. We also advocate for access to world class medical care and modern medicines.

Our team of social workers visit and support families in their homes and medical settings. We provide parent-to-parent support, vouchers and allowances during hospitalisations, welfare assistance during hardship, medical equipment, assistance towards organ transplant costs and, when necessary, end of life support.

Cystic Fibrosis NZ also supports a comprehensive data registry containing clinical and other information about those with CF in New Zealand. The data registry provides an invaluable source of information to support assessment of health needs and applications for funding of medicines. [1]

Cystic Fibrosis NZ receives less than 4 per cent government funding and relies on the generosity of New Zealanders to meet the ongoing needs of our community.

CONSULTATION

In preparing this submission, Cystic Fibrosis NZ has drawn on the wealth of information provided by those with living with CF, their families and whānau, and the expert clinicians who understand and treat CF.

RECOMMENDATION

Recommendations made by this submission.

Cystic Fibrosis NZ understands that it is not the role of the Health Select Committee to make decisions about which medicines are funded in New Zealand.

We are therefore asking the Committee to do all it can to help secure public funding of Trikafta in New Zealand for all those with CF who would benefit, by:

- Urging Government to provide Pharmac with sufficient budget available to fund Trikafta.
- Urging Government to make significant improvements to medicines' funding arrangements to provide wider access to medicines for New Zealanders, in particular access to modern medicines for those with disabilities and rare disorders.
- Considering the findings and recommendations of the independent Pharmac Review Panel as soon as its Final Report is released; and
- Supporting significant improvements to the arrangements for the funding of medicines as part of the finalisation of the restructured health system to be in place from 1 July 2022.

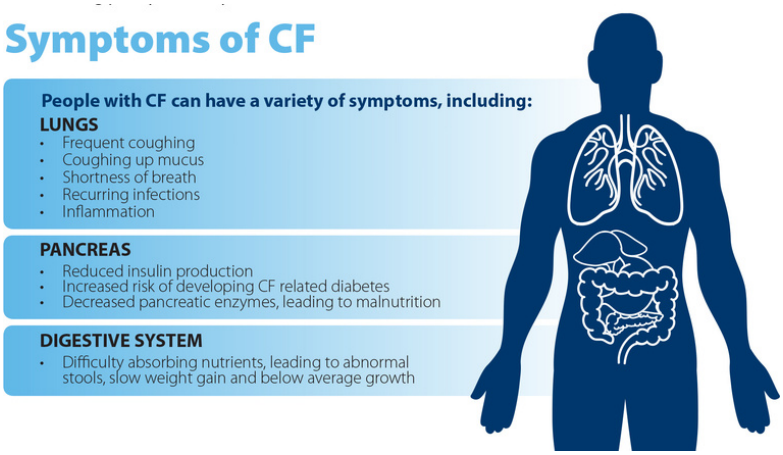
SECTION 1 DISCUSSION

1.1 New Zealand's CF Community

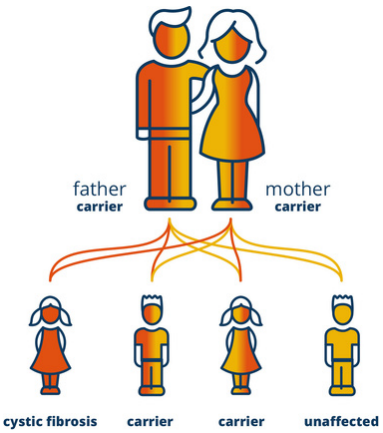
- There are more than 540 people in New Zealand with CF.
- The CF population is much younger than the NZ population, with approximately 44 percent under the age of 16 years and 88 percent under 40 years of age, compared with 19 percent and 53 percent respectively for the NZ population overall.
- The average age of a person with CF is 18 years compared with 37 years for New Zealanders as whole.
- These differences are due to the lower life expectancy of those with CF, with only half of those with the condition reaching the age of 31 years compared with 80 years for NZ as a whole.
- Around 9 percent of those with CF in New Zealand identify as Māori. [2]
- Many families have more than one family member with CF. Cystic Fibrosis NZ is aware of at least 53 families who have more than one child with the condition, with one family having three and another family having five children. Seventeen of these families have lost more than one child with CF.
- There are people with CF living in all parts of New Zealand, with around 30 percent living in the South Island and 70 percent in the North Island.
- Approximately 40 percent live in the four main centres.

1.2 What is CF?

CF is an inherited genetic condition that causes the body to produce thick, sticky mucus resulting in serious clinical consequences for multiple organs in the body, including the lungs, pancreas, liver, digestive and reproductive systems. It is the most common life-threatening genetic condition in this country.



1.3 How does someone get CF?



25 percent (1 in 4)
the child will have CF

50 percent (1 in 2)
the child will be a carrier but will not have CF

25 percent (1 in 4)
the child will not be a carrier of the gene and will not have CF

CF is a recessive genetic condition with a person with CF inheriting two damaged or mutated copies of the CF gene – one from each parent. In New Zealand one person in 25 carries the cystic fibrosis gene, usually without knowing it.

Carriers do not have symptoms of CF and most people are not aware they carry the CF gene unless there is a family history of the condition. If two CF carriers have a child together, there is a 1 in 4 or 25 percent chance that the baby will be born with CF.

In New Zealand, CF is generally identified through the heel prick test undertaken on newborn babies and confirmed through sweat chloride and gene testing.

There are a substantial number of CF gene mutations that cause CF, with the most commonly occurring being the F508del mutation. Approximately 90 percent of people with CF in New Zealand carry at least one copy of the F508del mutation.

1.4 Life with CF under current treatments

Of the more than 540 people with CF in New Zealand, only a small proportion have access to medicines that treat the cause of the condition.

- Approximately 35 people with CF have access to Kalydeco, currently funded by Pharmac for those with a specific CF gene mutation.

- A very small number have been able to fund Trikafta privately or received it through Vertex's Managed Access Programme. This Programme only provides Trikafta for those in critical need.

The remaining majority have access to a treatment regime that addresses only the symptoms of CF. This regime commences on diagnosis and continues throughout life with CF, increasing in scale and intensity as complications arise, the condition becomes more severe, and health declines.

Current treatment and medication help to manage the effects of the condition, but do not ultimately prevent the complications, decline and premature death of almost all of those with CF.

Base treatment Regime

New parents spend the early days of their child's life attempting to come to terms with the diagnosis and learning how to manage a serious and complex condition.

Early treatment can be complicated when a baby has an intestinal blockage that occurs in about 20 percent of newborn babies with CF. This is treated through medical or surgical means and results in additional time in hospital.

Family and whānau then must take on responsibility for managing the treatment regime which continues throughout the life of a person with CF and comprises:

- lung clearance
- exercise
- medication
- nutrition
- protection from infection

Percussion is used on babies, toddlers, and young children up to the age of 5 or 6 years. It is carried out by parents or caregivers and done twice daily to try to clear the thicky, sticky mucus from their lungs.

From 5 or 6 years of age, children are taught how to perform physiotherapy with a Positive Expiratory Pressure (PEP) technique using a PEP device, sometimes with a nebulised saline solution.

By the age of 10 or so, some children may be nebulising medication to help to thin and clear mucus from their lungs, in addition to PEP and saline.

Most people with CF have impaired pancreatic function and so are unable to digest food and absorb some vitamins and minerals. As a result, they need to take pancreatic enzymes with every meal or snack. Almost 80 percent of people with CF require pancreatic enzymes and forgetting to take them can lead to abdominal discomfort, diarrhoea, and inability to increase their weight.

Nutrition:

- 150% of a normal calorie intake is needed in a diet for people with CF due to malabsorption and the demands of fighting infection.
- Healthy weight is critical to help to maintain growth and development in children and preserve lung function.
- Around half of those with CF under 16 years of age require some form of supplemental feeding to help maintain their weight.

Infections:

- Protection from respiratory infections is extremely important and can have a significant impact on daily life.
- It is recommended that babies and children with CF do not attend a general day care in the early years of life, and that social situations and crowded places where there may be those with coughs and colds are avoided.
- People with CF are also strongly discouraged from mixing with others with CF due to the risk of cross-infection of lung bacteria.

Clinical management:

- People with CF who have not developed significant complications have a three-monthly outpatient appointment at their closest hospital.
- They also undergo an annual review where a more comprehensive assessment is undertaken including chest x-ray, ultrasound, bone scans, blood tests, and oral glucose tolerance tests, servicing of PEP and nebulising equipment, and consultations with the CF consultant, physiotherapist, dietician, nurse and, for adults, may also include a psychologist.

- Those living outside main centres, in particular adults, may need to travel significant distances to reach their appointments adding significantly to time taken for appointments and can require overnight stays. For example:
  - adults with CF in Northland must travel to Auckland
  - adults and children from the West Coast need to travel to Christchurch

1.5 CF Complications

Over time, complications arise and the health of those with CF almost inevitably declines although complications can occur at any age, and all require additional treatment over and above the base CF regime.

People with CF may experience problems with other organs:

The most commonly experienced impacts of CF are:

Increasingly frequent and chronic lung infections

- The thick, sticky mucus produced by those with CF is difficult to cough up, trapping bacteria, viruses and fungus in the lungs and causing increasingly frequent infections.
- These cause inflammation, lung damage and chronic infections, declining lung function, increasing breathlessness, fatigue, and a chronic cough.
- At first, treatment is with oral antibiotics but, over time, and depending upon the nature of the infection, treatment is with nebulising antibiotics and intravenous antibiotics (IV).
- Inpatient stays for 14-day hospital stays are usually required for IV antibiotics to be administered.
- As those with CF experience increasing lung infections, IV treatments are needed more frequently.
- Some people with CF require permanent access for IV antibiotics delivered through a port implanted in the chest.

CF-related diabetes

- The thick, sticky mucus causes scarring to the pancreas, leading to insulin deficiency.
- The incidence of CF-related diabetes increases with age, with around a quarter of those over 16 years of age having the condition.
- It is usually managed with blood glucose monitoring and daily insulin injections.

Failure to gain and / or retain weight

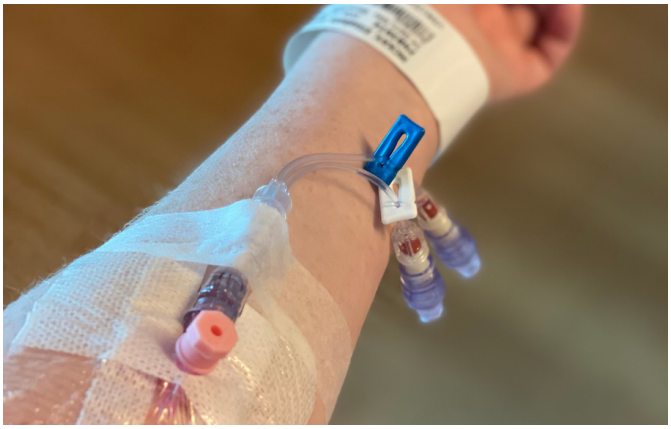
- Maintaining adequate weight can be challenging for those with CF as lung function declines and complications develop.
- Where oral supplements are no longer adequate to help maintain calorie intake, those with CF may have naso-gastric feeding when in hospital or may need to have a permanent tube inserted to provide direct access to the stomach for feeding, often overnight.

Other complications of CF:

As complications arise some people with CF will require more frequent hospital outpatient appointments, hospitalisations, regular monthly clinics and an annual review.

Some of these complications can include:

- complex liver disorders
- sinus problems requiring surgery
- low bone density
- gall bladder removal
- gastric reflux
- depression and anxiety
- bleeding from the lungs
- arthritis
- stress incontinence mostly in girls and women due to chronic coughing

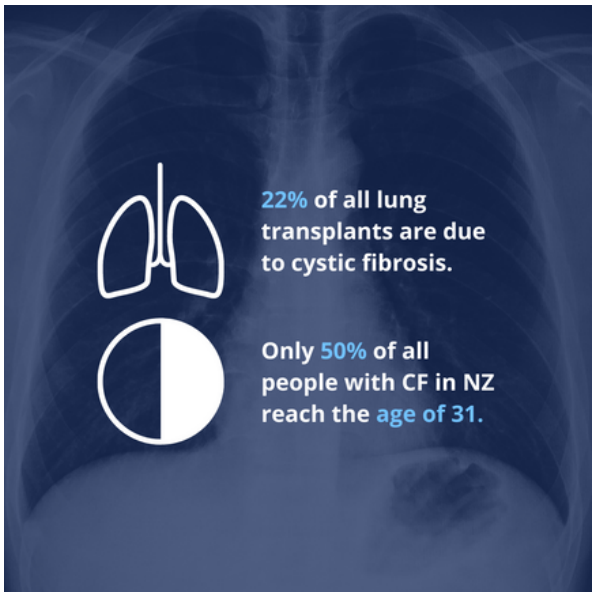


in 2018, over a third of people with CF had IV antibiotics in hospital, spending an average of 20.9 days as inpatients.

Treatment starts very young, with almost a quarter of those 4 years and under spending an average of 15.9 days in hospital having IV antibiotics. [3]

1.6 Seriously unwell including lung transplant

As lung infections increase, hospitalisations become increasingly frequent and lung function declines, people with CF may need oxygen therapy. Some may need to wear a mask at night to ensure that they have sufficient oxygen while sleeping, while others may have a machine in their home or need to take a portable tank with them when they travel.



At this stage, it is likely they have been considered for lung transplant. Assessment for transplant is usually initiated when a person's lung function has declined to around 30 percent of normal levels, frequent IV antibiotics are needed, and they are struggling to maintain weight. Clinicians may consider that a person may have only two or three years to live without the procedure.

Assessment for transplant involves a comprehensive range of clinical, physical and laboratory testing to determine suitability. The process is spread over a week and is undertaken in Auckland. Potential recipients usually have a support person with them during the process. Not everyone assessed is accepted: some may be deferred because they need to make changes such as gaining weight, while others may be declined because they are too unwell, there may be compliance issues, or other health reasons.

If accepted onto the active transplant list, there is no way to know how long each person might have to wait for suitable lungs.There can be false alarms, being called into hospital only to find that the lungs may not be suitable, or the transplant cannot proceed for other reasons. Some people die while waiting for suitable lungs.

Most people with CF will have double lung transplants, with the procedure taking between 8 and 12 hours. They then spend several days in ICU, moving to the High Dependency Unit, and then to a respiratory ward for several weeks depending upon progress. Patients then move to Hearty Towers at Green Lane Hospital in Auckland for around 6-12 weeks to recover and to learn to manage their new treatment and medication regime.

All transplant patients must have a support person with them for the full duration of the lung transplant process, including at Hearty Towers, meaning that a major commitment is needed from families and whānau for the procedure to take place. As lung transplants are only undertaken in Auckland, this is even more demanding on those living outside the region.

For those who are too unwell, or who are considered unsuitable for transplant, palliative care remains. With the aim of keeping the person with CF as comfortable as possible, this is likely to include extended hospital stays, continuing IV antibiotics, and permanent oxygen.

Recovery from transplant can take considerable time and involves comprehensive monitoring in the first year. Rejection can occur at any time, resulting in more frequent appointments, hospital stays, and increased monitoring. For some, retransplant may be needed.

Post-transplant

Survival rates for people with CF are around 90 per cent for the first year after transplant. More than 70 lung transplants have been carried out on people with CF in New Zealand since the procedure was first undertaken here in 1993. The median survival rate for those with CF since lung transplants were first undertaken in New Zealand is 5.7 years.

Lung transplantation is a traumatic and high-risk procedure, a last resort for those with CF who are approaching respiratory failure. Prior to therapies such as Kalydeco and Trikafta , transplant was the only available option to extend the life of someone with CF.

It has provided many people with CF with extra years which they would not otherwise have had. However, it is not a cure for CF with those who have had transplants swapping one treatment regime for another, and still having CF in their other organs.

1.7 Impact of CF on quality of life and wellbeing

CF progression can affect life in many ways

The current treatment regime for the majority of the people with CF in New Zealand has a major negative impact on those with the condition, their families and whānau as a result of undertaking daily treatment, attending hospital outpatient appointments, and spending time in hospital.

This burden grows heavier as complications develop, the health of the person with CF declines, and the treatment regime expands and intensifies, and is exacerbated where there is more than one family member with CF.

The impact includes the time and support needed to manage the treatment regime, loss of opportunities and income, and the financial costs of with living with CF. Living with CF also has a lifelong negative impact on mental well-being due to stress, isolation and loneliness, and the fear and grief of living with a serious illness and facing early death.

For babies, toddlers and younger children, the burden of the CF treatment regime is borne by parents. Following diagnosis and discharge from hospital, they must manage an ongoing routine of lung clearance, medication, nutrition, and hospital visits for their new baby, at the same time as coming to terms with having a child with a serious and life-limiting condition. Some parents have to give up their jobs to stay at home to look after children with CF, and it is usually the mother that does so.

As children grow and start to carry out lung clearance and take medication themselves, supervision and oversight is still needed to ensure that treatment is undertaken properly, and medication is taken. The burden of care continues to fall primarily upon parents and caregivers.

There are also challenges as children with CF want to participate in the same activities as their peers and friends. Many seemingly simple experiences, such as going on school camp and for sleepovers, can be difficult for children with CF due to the demands of their treatment and medication. Many children do not wish for others to know about their condition, and there is not always a suitable adult to take responsibility for supervising treatment away from home. This can be challenging for both parents and children - they want to participate but may not be able to adequately manage the risks.

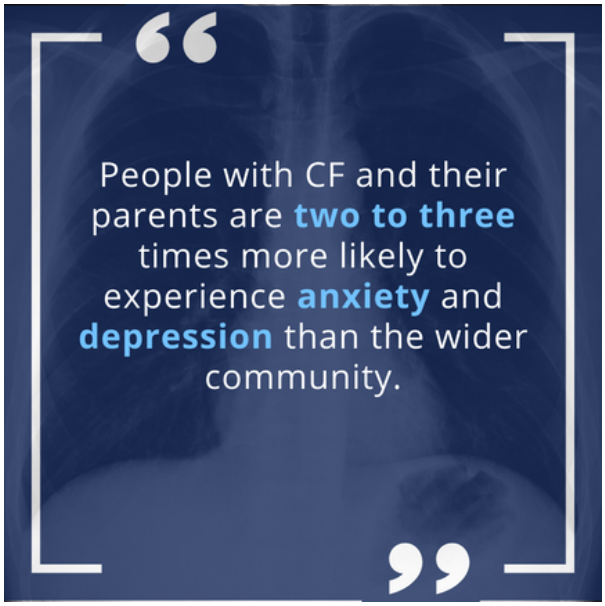
Increasing CF complications also mean more time away from school, more time in hospital, and more treatment, reducing opportunities to participate in sport and other activities. The burden on parents and whānau also increases as more time is needed for treatment, taking children and adolescents to hospital appointments, and managing hospital inpatient stays.

Dealing with inpatient stays can be particularly challenging, as they are generally for 14 days and, depending on the age and maturity of the child or adolescent, may require a parent or caregiver to stay with them for all or part of the time. Where the hospital is not in the same town or city, and where there are other children in the family to care for, the challenge is even greater.

By later adolescence and early adult years, those with CF are trying to come to terms with the reality of living with a serious and ultimately terminal condition. This is a time of significant challenge as they try to manage their condition, cope with increasing complications, spend more time on treatment, and deal with the emotional and psychological consequences of CF. For some, it provides the motivation to work as hard as they can to manage their treatment regime, take their medication, and keep as well as possible for as long as possible. Others take the view that there is little point in doing treatment, studying, having relationships, or planning for the future because they aren't going to have one.

As those with CF study and enter the workforce, it can be difficult to find the appropriate courses and employment. It can be very challenging to move away from home, due to the practical difficulties of managing a treatment regime and staying well without the day-to-day support of family and whānau. It can also be difficult to find employment that accommodates the demands of CF, and employers who are sympathetic and supportive to those with the condition. Hospital appointments and frequent inpatient stays quickly use up sick leave and holidays.

Family and whānau are likely to continue to play a significant role at this stage for those with CF who experience increasing complications and declining health. The person with CF may not be able to work or live alone, and may need significant support from their parents, partners, family and whānau to cope with their daily lives, for example, when a person with CF is waiting for suitable lungs for a transplant.



The impact of CF on the mental well-being of those with the condition, their families and whānau is immense and lifelong. Many people feel unhappy, depressed, and unable to overcome their problems. This is exacerbated where a family has more than one member with CF and they have already lost someone to the condition. They live with the grief arising from that death, as well as the fear that the same thing will happen again. Many also feel isolated from family and friends, and experience significant family tension due to CF. Living with a family member with CF can place significant strain on personal relationships, with a high rate of divorce and relationship breakdown amongst parents with CF children.

Those with CF, their families and whānau live every day with the expectation that they will face declining health, increasing complications, major medical intervention, and premature death. The emotional and psychological burden can be overwhelming, with many experiencing continuing fear about what the future will bring and how they will cope. Relief from this burden and the anxiety and grief which accompanies it, is what those with CF, their families and whānau continue to hope for.

SECTION 2 TRIKAFTA

2.1 What is Trikafta and what benefits would it have?

Trikafta is manufactured by Vertex Pharmaceuticals, a US biotech company. It was first approved for use in the United States by the Federal Drug Administration (FDA) in October 2019. [4]

It is a cystic fibrosis transmembrane conductance regulator (CFTR) or CFTR modulator therapy. It is designed to target the CFTR protein defects caused by the F508del mutation, the most commonly occurring CF mutation. It is a combination of three drugs (elexcaftor/tezacaftor/ivacaftor) which together help to improve the function of these proteins, making the mucus in the body more hydrated and easier to clear.

It is one of four CFTR modulator therapies now available for people with various CF gene mutations, all manufactured by Vertex. Only one of these four, Kalydeco which was approved by US FDA in 2012, is currently funded by Pharmac. Trikafta is funded in more than 30 countries, including the US, the UK and Australia.

Benefits of Trikafta

The benefits of Trikafta to those who carry at least one copy of the F508del mutation are very significant, bringing immediate, and enduring improvements by increasing lung function and weight gain, and reducing the burden of treatment.
Real world data from more than 16,000 US patients treated with Trikafta showed. [5]:

Trikafta Real-world data

Sourced from >16,000 U.S. patients treated with TRIKAFTA

- **87%** Reduction in risk of lung transplant*
- **77%** Fewer pulmonary exacerbations**
- **74%** Reduction in risk of death***

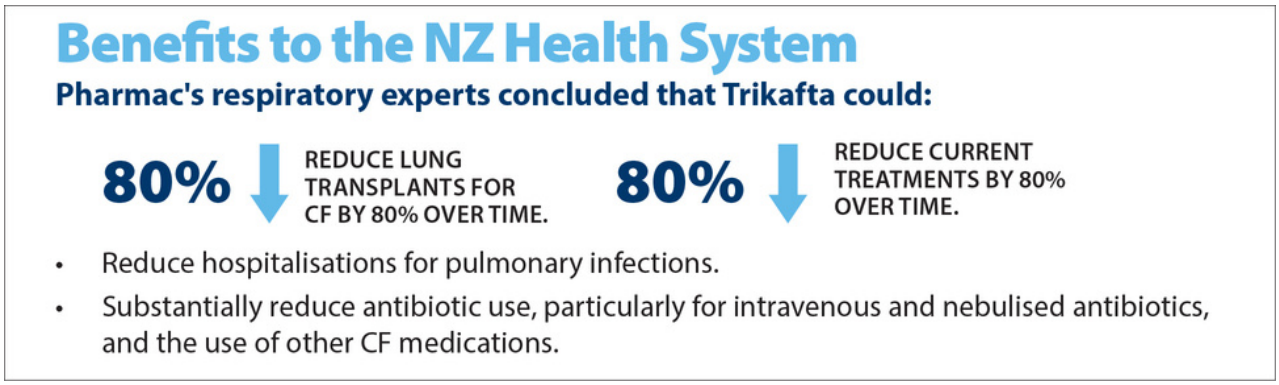
* Unadjusted estimate relative to historical 2019 U.S. Cystic Fibrosis Foundation registry data for patients older than 12 with at least one copy of F508del mutation

** Relative to 12-month period prior to TRIKAFTA treatment initiation Data from observational post-authorisation safety study on >16,000 TRIKAFTA-treated patients with mean of ~9 months of exposure, from U.S Cystic Fibrosis Foundation patient registry.

Pharmac's expert clinical advisers, who assessed Trikafta, called it a paradigm-shifting treatment for CF in that it treats the cause of the condition. They concluded that it provides patients with substantial improvements in quality of life and an expectation of substantial prolongation of life, as well as allowing people with CF to live a nearly normal life.

They 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.

Funding Trikafta would have significant wider benefits.



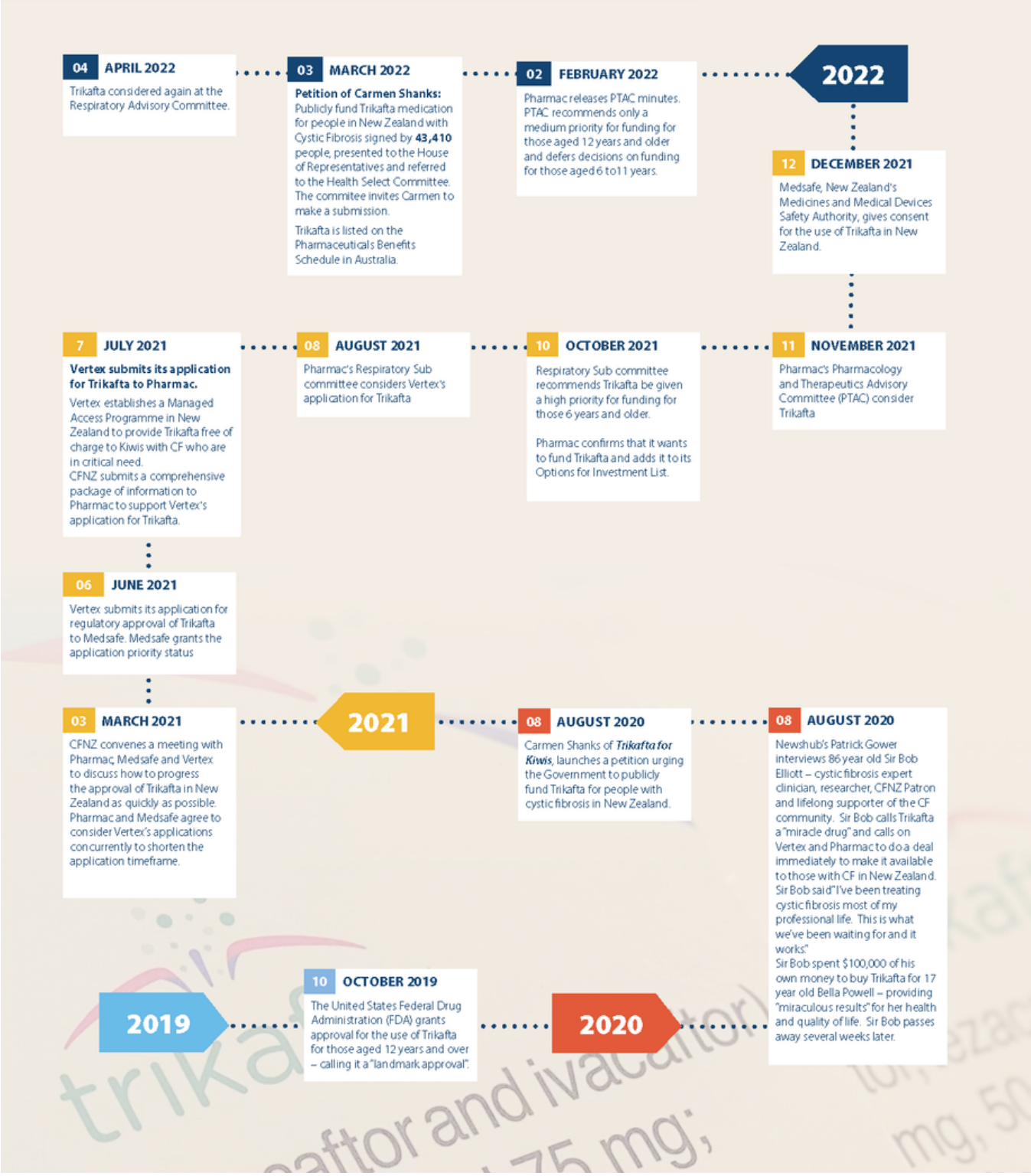
Pharmac's respiratory experts estimated that Trikafta would benefit 388 people with CF aged 6 years and over, over 70 per cent of those in New Zealand with the condition. [6]

Trikafta provides significant, immediate, and enduring improvements:

- Positive financial and economic impacts for those with CF, their families and whānau
- Enabling many to be able to complete their education, seek employment or return to work
- Reducing the substantial burden of time and care required for treatments
- Substantial and meaningful improvements to quality of life
- Increase lung function
- Weight gain
- Increasing life expectancy
- Improvement in symptom burden for both those with progressed disease and younger patients who have not yet developed significant organ damage or lung impairment

One of the greatest benefits would be relieving the fear and anxiety currently faced by those with CF, their families and whānau, together with the pleasure of finally being able to plan for a positive future.

2.2 Current status of Trikafta in Aotearoa



Medsafe

In June 2021, Vertex applied to Medsafe for consent for the use of Trikafta in New Zealand.

It is Medsafe's role to assess medicines to ensure that they meet acceptable standards of safety, quality and efficacy before they can be made available in New Zealand.

Medsafe granted Vertex's application priority status. Priority status is granted on the basis of significant clinical advantage or significant potential cost savings to the taxpayer.

Medsafe granted consent for the use of Trikafta in December 2021, less than six months from submission of the application.

Application to Pharmac

In July 2021, Vertex applied to Pharmac for funding of Trikafta for the treatment of cystic fibrosis patients aged 6 years and older with at least one copy of the F508del mutation.

Pharmac referred the application to its expert clinical advisers at the meeting of the Respiratory Subcommittee held on 26 August 2021.

In October 2021, Pharmac released the minutes of the Respiratory Subcommittee's meeting who recommended that Trikafta be listed with a high priority for funding for those with CF aged 6 years and older.

In making this recommendation, the Subcommittee noted:

- The significant health need for those with CF aged 6 years and older for whom there are no funded CFTR modulator therapies
- The strong evidence of benefit of Trikafta in those with at least one F508del mutation in the CF gene. [7]

At the same time as releasing the Subcommittee's minutes, Pharmac announced that it had undertaken an economic health assessment and determined that Trikafta is a medicine that it wants to fund. Pharmac had also added Trikafta to its Options for Investment list. Pharmac also advised that it intended to take further advice from its Pharmacology and Therapeutics Advisory Committee (PTAC) to help determine where the application should be ranked against the other medicines it wants to fund. [8]

PTAC met in November 2021 and Pharmac released PTAC's recommendations in February 2022.

- Contrary to the findings of the Respiratory Subcommittee, PTAC recommended funding Trikafta with only a medium priority for those aged 12 years and over and deferred a decision for those under 12 years of age.
- PTAC noted in making its recommendations that there was a lack of longer-term evidence of benefit, and insufficient evidence supporting efficacy in those aged under 12 years. [9]

Cystic Fibrosis NZ is seriously concerned at PTAC's assessment and recommendations and wrote to the Chair of Pharmac to express its concerns.

Pharmac's announcement in October 2021 had raised the hopes of the CF community, only to dash them some five months later, with a particularly devastating impact on those with children aged 6-11 years. It was also difficult to understand how PTAC could reach such different conclusions to Pharmac's expert clinical advisers, including those who treat CF and have seen the effects of Trikafta first-hand.

Cystic Fibrosis NZ asked Pharmac to seek reconfirmation from the Respiratory Subcommittee of its recommendation of high priority for funding of Trikafta for all those aged 6 years and over; to negotiate a fair and reasonable price for Trikafta with Vertex; and, to consider a decision to fund Trikafta as soon as it receives its Budget allocation for 2022/23 and beyond.

Pharmac responded by advising that PTAC's recommendations do not override those of the Respiratory Subcommittee and that the advice was complementary. However, the response noted that Pharmac was not able to provide a definitive timeframe for if, or when, a decision would be made to fund Trikafta.

Application to Pharmac - next steps

Cystic Fibrosis NZ understands that further advice will be sought from Pharmac's Respiratory Advisory Committee at its meeting on 27 April 2022. PTAC is expected to consider the Trikafta applications and further advice at its meeting on the 19th and 20th of May 2022.

SECTION 3 NEED FOR CHANGE

3.1 Need for change to funding medicines in Aotearoa

Cystic Fibrosis NZ considers that there is an urgent need for change to the arrangements for funding medicines in New Zealand, in particular for modern medicines that treat rare disorders such as cystic fibrosis.

A significant increase in medicines' funding is needed, together with improvements to the way such medicines are assessed and prioritised for funding.

NZ lags behind comparable OECD Countries

Analysis by Medicines New Zealand of New Zealand between 2011-20 compared with 19 other OECD countries showed that New Zealand lags behind most comparable countries in access to modern medicines, and that public funding of modern medicines is significantly slower than in comparable countries.

New Zealand ranks last of 19 OECD countries for the number of publicly funded modern medicines, funding 34 compared with 120 in Australia, 183 in Finland, and 251 in Great Britain. The average time taken from in-country registration to public funding in New Zealand exceeded two years, almost double the average for the OECD20 countries, and the second longest time amongst the OECD20 countries. [10]

3.2 Independent review of Pharmac

The Government last year acknowledged public concerns over how medicines are assessed and funded by commissioning an independent review of Pharmac. The purpose of the review was to assess:

- how well Pharmac is performing against its current objectives and whether and how its performance could be improved; and
- whether its current objectives maximise its potential to improve health outcomes for all new Zealanders as part of the wider health system, and whether and how these should be changed. [11]

The Pharmac Review Panel's Interim Report was publicly released by the Minister of Health in December 2021. The Panel's initial assessment concluded that:

- Pharmac is underperforming in helping to remove inequitable health outcomes.
- Its prioritisation approach appears to disadvantage Māori, Pacific people, disabled people and those with rare disorders.
- Te Tiriti o Waitangi principles are largely unseen in decision-making processes.
- There may be an excessive focus on containing costs - and a concern the cost-saving model may not be the right one to meet future health needs.
- Decision making is opaque and is perceived as being slow.
- Engagement with consumers and patient advocacy groups needs to be more meaningful.
- Convoluted procurement processes put off pharmaceutical companies.
- A perception New Zealand is falling behind other developed countries. [12]

Cystic Fibrosis NZ's experience is consistent with the conclusions of the Review Panel, in particular the excessive focus on containing costs and that the prioritisation approach disadvantages those with disabilities and rare disorders.

At the time of writing, the Pharmac Review Panel's Final Report had not been released. Cystic Fibrosis NZ hopes that this will happen very soon, in order to ensure there is full and transparent consideration of the Review Panel's recommendations prior to the finalisation of the arrangements for the restructured health system to be in place from 1 July 2022.

3.3 Health Select Committee - Kalydeco Petition

In November 2019, the Health Select Committee received a petition from Edward Porter seeking public funding for the medication Kalydeco to treat CF for those with a specific genetic mutation. The Committee received submissions from Edward Porter and Cystic Fibrosis NZ seeking public funding of Kalydeco, and raising issues over Pharmac's budget, resourcing, and processes. In Cystic Fibrosis NZ's view, these issues contributed to the long delay in making Kalydeco available, with Pharmac taking six years to assess and fund the medicine.

The Committee released its report on the petition in March 2021, noting that Kalydeco had been funded by Pharmac in March 2020. The Committee noted that the issues raised would be addressed through the Pharmac review and stated that "we intend to monitor the progress of the review closely and looks forward to its final outcome." [13]

We therefore ask the Committee consider the findings and recommendations of the independent Pharmac Review Panel as soon as its Final Report is released.

3.4 Pae Ora (Heathy Futures) Legislation

Cystic Fibrosis NZ understands that the Pae Ora Legislation Committee will shortly report back to Parliament with its recommendations in respect of the legislative arrangements for the restructured health system. Cystic Fibrosis NZ made a submission on the Pae Ora (Health Futures Bill) seeking, amongst other things, significant improvements to the proposed arrangements for the funding of medicines under the new system.

Cystic Fibrosis NZ asks the Committee support significant improvements being made to the arrangements for the funding of medicines as part of the finalisation of the restructured health system.

CONCLUSION

CF is cruel condition, imposing a life-long burden upon those with the condition, their families and whānau. They live every day with the expectation that they will face declining health, increasing complications, major medical intervention, and premature death. The emotional and psychological burden is overwhelming, bringing fear about what the future will bring and how they will cope.

Trikafta is a highly effective medicine that would relieve this burden for the majority of those with CF in New Zealand, allowing them to live almost normal lives. It would bring significant benefits to the NZ health system, and financial and economic benefits not only to those with CF but also to the wider community. Trikafta is already funded in more than 30 countries - New Zealand needs to be next. Kiwis with CF don't have time to wait.

Yours sincerely

Lisa Burns
Chief Executive | Cystic Fibrosis NZ

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