CYSTIC FIBROSIS NZ

2022

Advocacy Kit

#27MOREYEARS

GET TRIKAFTA FUNDED

TAKE ACTION

Join the #27MoreYears Movement

You're ready to help us in the #27MoreYears movement and step up our fight to get Trikafta publicly funded in New Zealand.

We need as many people as possible to join, make some more noise and help us fight until someone finally pays attention.

After the recent news that Trikafta is not being prioritised, the purpose of this toolkit is to provide you with the resources to help you take action to support the #27MoreYears campaign. **The focus is to put pressure on the Government and Pharmac.**

There are three key actions you can take today to help:

- **Write**
  - Send a letter to your local MP.
  - Send a letter to Pharmac.
- **Meet**
  - Request a meeting with your local MP and share your personal story.
- **Share**
  - Tell your story, contact your local media or let CFNZ know if you're would like to share your personal experience.

No matter how you choose to get involved, you can be a part of the fight to get Trikafta.

Your help means we are taking steps to help protect Kiwis from the cumulative and irreversible damage that happens over time from CF and lessen the decline in the health of so many.

As always we're here to help if you need us.

WHAT CAN YOU DO?

Raise your voice and share your story.
The power of real, heart-felt and moving stories that can influence decision-makers shouldn't be underestimated.
Make a short video to tell your story and share what Trikafta would mean to you and why.

Contact your local MP, write, request a meeting, or hold an event. Ring their local office or send an email and request a time to meet. You can find details here: **https://www.parliament.nz/en/get-involved/have-your-say/contact-an-mp/**

Write a letter to Pharmac using the key messages on page 4 and letter writing tips on page 5.

Connect CFNZ with anyone who has influence, or high-profile Kiwis that can help share and promote the campaign.

Help us spread the word to your networks - Like! Comment! Share!
When you see a news article or social post about CF or getting access to Trikafta, make sure you engage with it.
If you've written to your MP or Pharmac, share it and tag them in the post. **Use the #27MoreYears and tag @CysticFibrosisNZ**

Buy a piece of CruelNeedsKind merch
from **www.cruelneedskind.co.nz**, write the words #27MoreYears on a piece of paper and take a photo then send it to comms@cfnz.org.nz.
Keep an eye out for our new merch coming soon!

KEY MESSAGES

1

27 YEARS

Pharmac estimates that Trikafta would provide an additional 27 years of full health - almost doubling life expectancy for people with CF in New Zealand, only half of whom currently reach 31 years of age.

2

PHARMAC STEALING 10,456 YEARS

27 years for each of the 388 people who would benefit. We need a medicines framework that recognises the value of medicines like Trikafta.

3

TRIKAFTA NOT RANKED HIGH ENOUGH TO FUND

Pharmac has advised CFNZ that, following its September 2022 ranking meeting, Trikafta has not moved far enough up its Options for Investment List to progress funding at this time.

4

MORE THAN 30 COUNTRIES

Trikafta is now available in more than 30 other countries around the world including the US, UK, Canada, France, Spain, Germany, Ireland and now Australia.

5

HIGHLY COST EFFECTIVE

Trikafta is an investment in the people of New Zealand and our health system. The estimated annual social, economic and financial cost of CF is $116 million. Funding Trikafta would cost substantially less than $60 million per annum.

6

THE PHARMAC PROCESS

The failure of a medicine as effective as Trikafta to rank highly enough to be funded makes very clear Pharmac's process does not work for modern medicines that treat rare disorders like cystic fibrosis.

7

MINISTER OF HEALTH PROMISE

The Minister of Health promised in June 2022 that things will now change, stating that "getting the cheapest drugs isn't the only health strategy" but Pharmac doesn't appear to have got that.

8

PHARMAC'S APPROACH

Pharmac's approach is out of step with the rest of the world, it remains cost-focused and obsessed with procuring cheap drugs at the expense of health outcomes.

WRITE A LETTER

Tips for the structure of your letter

- How does CF affect you personally, or your family and whānau.
- What impact does the delay in funding Trikafta have on you.
- What would Trikafta mean to you and how would your life change.
- Local MP - How can you help me to make change?
- Pharmac - What are you doing to help and support change when it comes to funding modern medicines.

Letter writing tips

- If writing to Pharmac, also cc in the Minister of Health and opposition Health spokespeople.
- Remember you are trying to influence, don't use language that's sarcastic, judgmental or questions their motives.
- Make it easy to read, avoid using exclamation marks, heavy underlining, italics, or bold font.
- Keep it short and to the point.

QUESTIONS & ANSWERS

To some of the common questions

Below are some tips that may help when answering questions you might get asked by your local MP, or media.

What are you asking of me, what can I do to help?

- The current medicine's funding system needs urgent change and as an MP this is something you can help to influence. Ask questions like:
- Why didn't Trikafta cut it when the evidence is so compelling?
- Why is Pharmac spending so little investment on medicines for rare disorders?
- Does the case for investment in Trikafta not make it worthwhile for the return of benefits to our overall health system.

What's the main issue with this Trikafta application?

- Budget and the pharmac process doesn't work for modern medicines like Trikafta.
- Pharmac is cost focused and obsessed with procuring cheap drugs for the masses at the expense of health outcomes. That's not Trikafta.

Where is Trikafta funded?

- Trikafta is now funded in more than 30 countries around the world.
- The US, UK, Ireland, Canada, Germany and now Australia,
- Just about everywhere except NZ, maybe they know something we don't.

What's the diversity of the CF community?

- Approximately 10% of our community are Maori and Pacific, and cystic fibrosis fits the definition of rare disorders so when Pharmac says they aren't cost focused, they look at the health benefits and want to focus on equity - where is the evidence of that?

What is the estimated cost of cystic fibrosis?

- **CFNZ commissioned a piece of work that estimated the annual social, economic and financial cost of CF to be $116m. This information has been shared with Pharmac.**

What is the anticipated cost of Trikafta?

- We understand the cost of Trikafta would be significantly less than $60m.

What's the next step to move the application forward?

- The next opportunity to have Trikafta reviewed will be the next Pharmac Ranking Meeting held in December.
- CFNZ has been advised that for Trikafta to move up the Options for Investment List one of three things needs to happen:
  - More information that could change Pharmac's Factors for Consideration
  - A change in the price offered by Vertex
  - Additional budget uplist from the Government.

What are the current treatments available to people with CF in New Zealand?

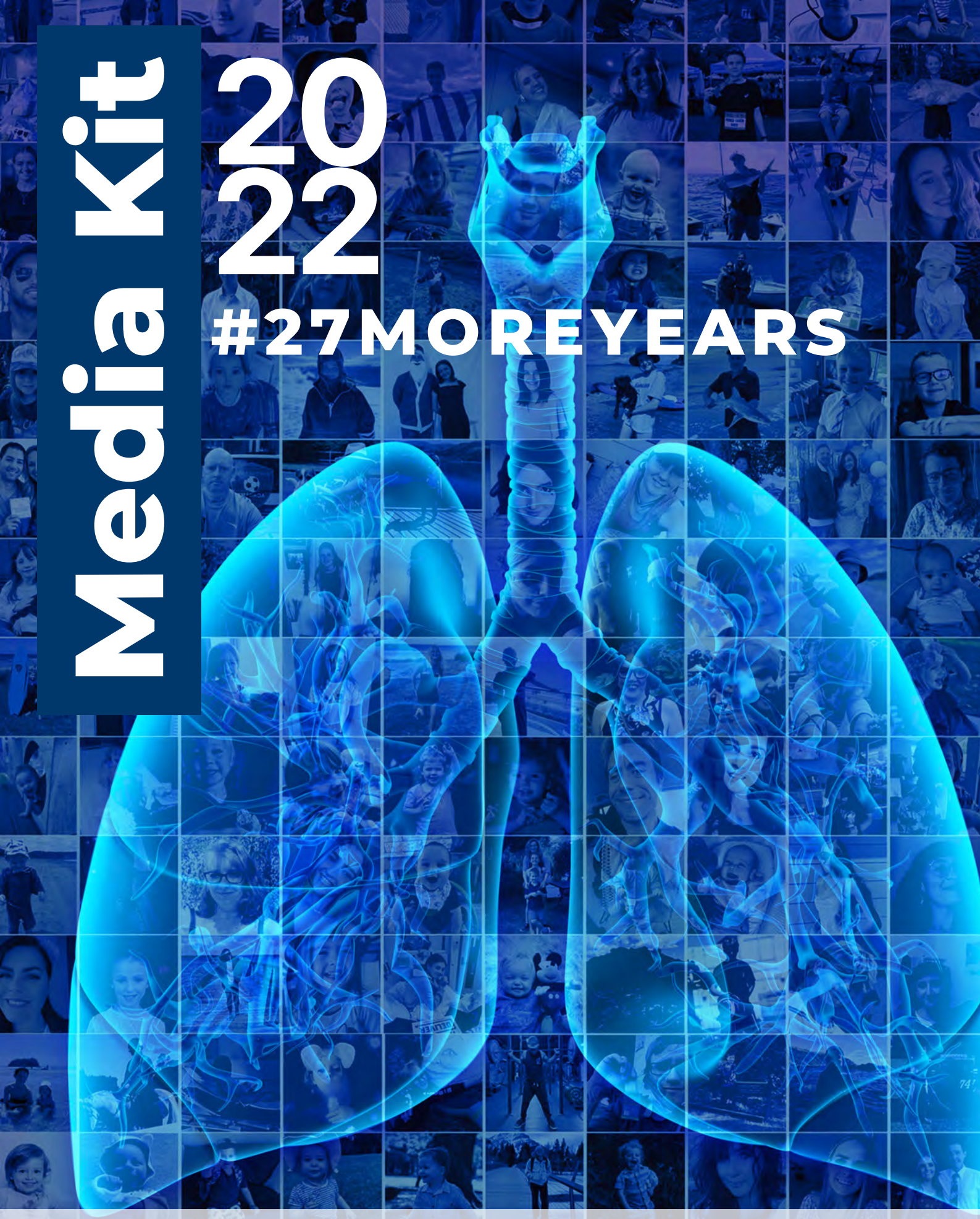
- Nebulising or essentially sucking on salt water / antibiotics
- Current treatments essentially are to treat symptoms, they don't stop the decline and they don't treat the cause.

Has CFNZ written to or been in contact with the Minister of Health?

- Yes, CFNZ wrote to the Minister in June after the budget announcement, they requested that in his letter of expectation to Pharmac that he outline medicines for rare disorders should be prioritised. To date they have not received a response.

Media Kit 2022

#27MOREYEARS



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CYSTIC FIBROSIS NZ

ABOUT CYSTIC FIBROSIS

CF is an inherited lifelong genetic condition that affects around 570 people in New Zealand, and is the most common life-threatening genetic condition in this country.

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

CF causes the body to produce thick, sticky mucus which damages the lungs, digestive system, liver and other parts of the body. People with CF endure a life-long, demanding and time-consuming daily regime of lung clearance and medication together with regular medical check-ups and, over time, increasingly frequent hospital admissions.

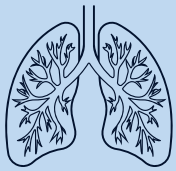
Only a very small number currently have access to CF modulator therapies that address the underlying cause of the condition. The remainder only have access to treatments that address the symptoms of CF.

The current treatment regime for CF commences on diagnosis and continues throughout life, increasing in scale and intensity as complications arise, the condition becomes more severe and health declines. While the health of those with CF generally declines as they get older, the complications arising from CF can occur at any age.

When deterioration becomes severe, a lung transplant may ultimately be the only option, but it is not guaranteed and it is not a cure. CF cuts short the lives of those with the condition, with only **half of those with CF in New Zealand reaching 31 years of age.**

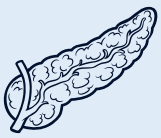
SYMPTOMS OF CF

People with CF have a variety of symptoms including:



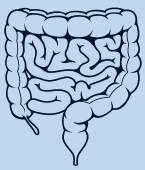
LUNGS

- Frequent coughing
- Coughing up mucus
- Shortness of breath
- Recurring infections
- Inflammation



PANCREAS

- Reduced insulin production
- Risk of developing CF related diabetes
- Decreased pancreatic enzymes, leading to malnutrition



DIGESTIVE SYSTEM

- Difficulty absorbing nutrients, leading to abnormal stools, slow weight gain and below average growth

People with CF may also experience problems with other organs such as their:

Liver

Reproductive Organs

Sweat Glands

Sinus

Gastrointestinal Tract

IMPACT OF CF

UP TO

40

tablets a day

MORE THAN

2

hours of physiotherapy and nebulising a day

22%

of all lung transplants are due to CF

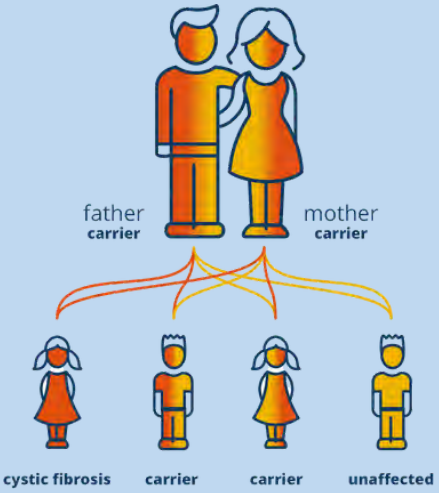
50%

Only half reach 31 years of age.

CF PROGRESSION CAN AFFECT LIFE IN MANY WAYS

- Extended hospital stays
- Reduced productivity
- Mental health
- Family life
- Social relationships / interactions

HOW DOES A PERSON GET CF?



It's a cruel twist of fate that brings CF to newborn life.

Two people who by ill luck, carry the faulty gene without knowing it, which creates a new life with CF.

Carriers do not have symptoms of CF, and most people are not aware they carry the faulty gene unless there is a family history of the condition.

If two carriers have a child together, there is a 25 percent chance they will have a child with CF.

1 in 4
the child will have CF.

1 in 2
the child will be a carrier but will not have CF.

1 in 4
the child will not be a carrier of the gene and will not have CF.

There are a significant number of gene mutations that cause CF with the most commonly occurring being the F508del mutation.

Approximately 90% of people with CF in New Zealand carry at least one copy of the F508del mutation.

Trikafta targets the protein defects caused by the F508del mutation.

TRIKAFTA

- Trikafta (elexacaftor / tezacaftor / ivacaftor) is manufactured by Vertex Pharmaceuticals. It was approved by the US Federal Drug Administration in October 2019.
- Trikafta treats the underlying cause of CF and works for around 90% of those with cystic fibrosis.
- Trikafta is available to people with CF in more than 30 countries.

BENEFITS OF TRIKAFTA FOR PEOPLE WITH CF

TRIKAFTA PROVIDES SIGNIFICANT IMMEDIATE, AND ENDURING IMPROVEMENTS:

- Increasing lung function.
- Weight gain.
- Reducing the burden of treatment.
- Substantial improvements to quality of life.
- 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.

PHARMAC'S RESPIRATORY EXPERTS CONCLUDED THAT TRIKAFTA COULD:

- Reduce current treatments by 80% over time, including hospitalisations for pulmonary infections and lung transplants.
- Substantially reduce antibiotic use, particularly for intravenous and nebulised antibiotics, and the need for dornase alpha, hypertonic saline, and chest physiotherapy.
- Allow people with CF to live almost normal lives.

27 YEARS

The number of years Pharmac estimates that Trikafta can add to the life of a person with CF.

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.

TRIKAFTA TIMELINE

2019

OCTOBER 2019

The United States Federal Drug Administration (FDA) grants approval for the use of Trikafta for those aged 12 years and over - calling it a "landmark approval".

2020

AUGUST 2020

Newshub's Patrick Gower interviews 86 year old Sir Bob Elliot - CF expert clinician, researcher, CFNZ Patron and lifelong supporter of the CF community. Sir Bob calls Trikafta a "miracle drug" and calls on Vertex and Pharmac to do a deal to make it available to those with CF in New Zealand. Sir Bob said "I've been treating CF most of my professional life. This is what we've been waiting for and it works."
Sir Bob spent $100,000 of his own money to buy Trikafta for 17 year old Bella Powell - providing "miraculous results" for her health and quality of life. Sir Bob passes away several weeks later.

JUNE 2021

Vertex submits its application for regulatory approval of Trikafta to Medsafe. Medsafe grants the application priority status.

MARCH 2021

CFNZ convenes a meeting with Pharmac, Medsafe and Vertex to discuss how to progress the approval of Trikafta in NZ as quickly as possible. Pharmac and Medsafe agree to consider Vertex's applications concurrently to shorten the application timeframe.

2021

AUGUST 2020

Carmen Shanks of Trikafta for Kiwis, launches a petition urging the Government to publicly fund Trikafta for people with CF in NZ.

JULY 2021

Vertex submits its application for Trikafta to Pharmac.

Vertex establishes a Managed Access Programme in NZ to provide Trikafta free of charge to Kiwis with CF who are in critical need. CFNZ submits a comprehensive package of information to Pharmac to support Vertex's application for Trikafta.

AUGUST 2021

Pharmac's Respiratory Sub Committee considers Vertex's application for Trikafta.

OCTOBER 2021

Respiratory Sub Committee recommends Trikafta be given a high priority for funding for those 6 years and older. Pharmac confirms that it wants to fund Trikafta and adds it to its Options for Investment List.

NOVEMBER 2021

Pharmac's Pharmacology and Therapeutics Advisory Committee (PTAC) consider Trikafta.

DECEMBER 2021

Medsafe, New Zealand's Medicines and Medical Devices Safety Authority, gives consent for the use of Trikafta in New Zealand.

2022

FEBRUARY 2022

Pharmac releases PTAC minutes. PTAC recommends only a medium priority for funding for those aged 12 years and older and defers funding decisions on ages 6 to 11 years.

MARCH 2022

Petition of Carmen Shanks

Publicly fund Trikafta medication for people in NZ with CF signed by 43,410 people, presented to the House of Representatives and referred to the Health Select Committee. The committee invites Carmen to make a submission.

Trikafta is listed on the Pharmaceuticals Benefits Schedule in Australila.

APRIL 2022

Trikafta considered again at the Respiratory Advisory Committee

MAY 2022

Trikafta considered again at PTAC's May meeting.

JUNE 2022

Carmen Shanks and CFNZ present to the Health Select Committee in support of Carmen's petition to have Trikafta publicly funded.

AUGUST 2022

Pharmac releases its assessment of Trikafta, together with the record of PTAC's May meeting. PTAC recommends a medium priority for funding Trikafta for those aged 6 and over. Pharmac estimates that Trikafta would provide people with CF an extra 27 years of full health and reconfirms that they want to fund it.

AUGUST 2022

Pharmac releases the record of the Respiratory Advisory Committee's (RAC) April meeting. RAC again recommends a high priority for funding Trikafta for those aged 6 and over.

SEPTEMBER 2022

Pharmac advises CFNZ that, following its re-ranking meeting, Trikafta has not moved far enough up its Options for Investment List to move forward with funding at this time. Pharmac advises CFNZ that the "door is not closed" and again states that "Trikafta is a medicine we want to fund". They also advise CFNZ that changes will be needed to enable Trikafta to move up the OFI List but that, even if changes can be made, it will not happen before Dec 22.

MEDIA RELEASE
16 September 2022

Embargoed until 16 September 2022, 10.00 AM

PHARMAC SAYS IT CAN'T PROGRESS TRIKAFTA

Is **"HOPE"** the new medicine strategy for NZ?

Yet again, Pharmac has raised the hopes of Kiwis with cystic fibrosis only to shatter them.

After months of what appeared to be encouraging progress, Cystic Fibrosis NZ (CFNZ) has been advised by Pharmac that, following its September medicines' ranking process, Trikafta[1] has not moved up the Options for Investment (OFI) List.

The outcome of this is that Pharmac is not **in a position to move forward with funding Trikafta at this time,** and that funding is **unlikely to occur without changes to the information at hand.**

Lisa Burns, Chief Executive for CFNZ says "We are shocked and outraged at this decision from Pharmac. This highlights our significant concerns about what is going on within Pharmac, their processes, decision criteria, and how these decisions are being communicated. We have been left confused and don't have the words to convey our deep disappointment for our CF community."

In August 2022, Pharmac released the record of advice from its Pharmacology and Therapeutics Advisory Committee (PTAC) and published a summary of its Technology Assessment Report (TAR)[2] containing its assessment of the cost-effectiveness of Trikafta.

As part of that release, Pharmac acknowledged the major benefits that Trikafta offers, not just to the health system but an extension to life expectancy and quality of life, estimating that Trikafta would provide benefits equivalent to **27 more years at full health when compared to current treatments**.

Pharmac also stated that it had already re-ranked Trikafta on its Options for Investment (OFI) List and was continuing commercial discussions with Trikafta's supplier, Vertex, to try and reach an agreement that would enable funding.

However, Pharmac has now advised CFNZ that Trikafta was re-ranked again in September, but it has not moved high enough up the OFI List for it to progress to funding at this time. Pharmac has advised CFNZ that changes would be needed to one or more of the factors for consideration before Trikafta could be re-ranked again and have the chance to move further up the OFI List.

These have been outlined as:

1. More information that could change Pharmac's Factors for Consideration
2. A change in the price offered by Vertex
3. Additional budget uplift.

Lisa said "The failure of Trikafta to move up the OFI List to progress funding makes it very clear that the Pharmac process does not work for modern medicines. Despite all available evidence, assessments and reassessments, validation by their Clinical Experts, we're going round in circles with unnecessary delays due to poor process, and decision making."

"We are utterly thrown by Pharmac's decision not to prioritise funding Trikafta. It defies logic that a medicine with such positive impact doesn't make it up the ranking list for funding. The current average life-expectancy in New Zealand is 31 yet Trikafta could give Kiwis with CF an additional 27 years, and quality of life. It doesn't make any sense."

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[1] Trikafta is a medicine that treats the cause of cystic fibrosis for around 90% of those with the condition.
[2] https://pharmac.govt.nz/news-and-resources/news/2022-08-15-media-release-pharmac-shares-update-on-its-assessment-of-trikafta/

Pharmac stated in its August release that they don't just look at how much, in dollar terms a medicine costs, that they also consider the health benefits to the person taking the medicine and what the benefits would be to the healthcare system. This is inconsistent with the decision advised to CFNZ where cost appears to be a barrier in moving the application forward.

"Trikafta is now available in more than 30 countries around the world. Pharmac has had access to the same information and evidence, in fact they've had the benefit of all the additional worldwide clinical data provided by Vertex, so are they implying that all those countries got it wrong?" said Lisa.

Trikafta would be a highly cost-effective investment. Independent work commissioned by CFNZ for 2019/20, calculated the approximate annual social, economic, and financial **cost of CF to be $116m** which was shared with Pharmac. The estimated annual cost to **fund Trikafta would be significantly less than $60m**.

Pharmac has advised CFNZ that "the door is not closed" and that "Trikafta is a medicine we want to fund". But, even if changes can be made to enable Trikafta to move up the OFI List, Pharmac has advised that this **will not happen before December 2022**.

"In the meantime, those who can will make the decision to leave NZ, their home, families, education, and careers to access Trikafta or another CFTR modulator overseas. Others will desperately seek access to Trikafta by any means they can or try to access unregulated alternatives, potentially putting their health at further risk while simply trying to stay alive," said Lisa.

New Zealand's current medicines funding system is out of step with the rest of the world, cost focused and obsessed with procuring cheap drugs at the expense of health outcomes. The perception that the Pharmac model is the envy of other countries, is simply not true, it's an example of what not to do and their model hasn't been adopted anywhere else in the world.

"This game of chance, playing with the lives of our CF community is unacceptable. We need a medicines framework that recognises funding medicines like Trikafta is an investment in the people of New Zealand and our health system. Until we do, thousands of sick and vulnerable Kiwis will continue to experience needless suffering and early death."

"CFNZ and our CF community remains relentless in our determination to get Trikafta funded in New Zealand. **Kiwis deserve better than this, HOPE shouldn't be our only option for a medicine's strategy**."

- E N D S -

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For more detailed information please refer to the Cystic Fibrosis NZ media kit.