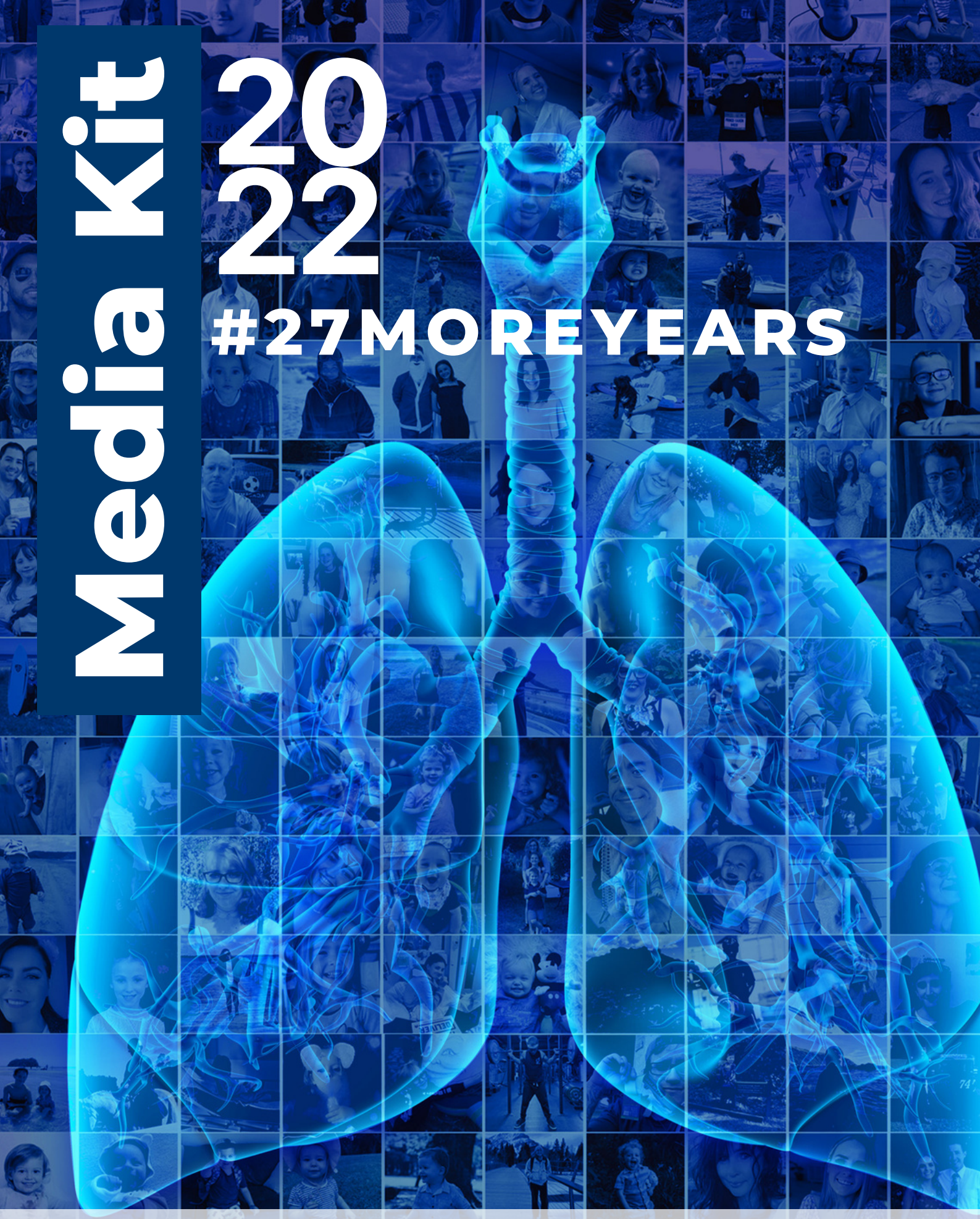
# Media Kit 2022

## #27MOREYEARS




(09) 308 9161

info@cfnz.org.nz

www.cfnz.org.nz

CysticFibrosisNZ

@CysticFibrosisNZ

cystic-fibrosis-nz


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.

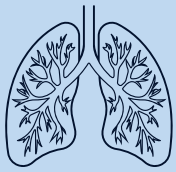
Only a very small number currently have access to CF modulator therapies that address the underlying cause of the condition. The remainder only have access to treatments that address the symptoms of CF.

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.**

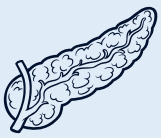
# SYMPTOMS OF CF

People with CF have a variety of symptoms including:



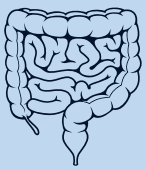
## LUNGS

- 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

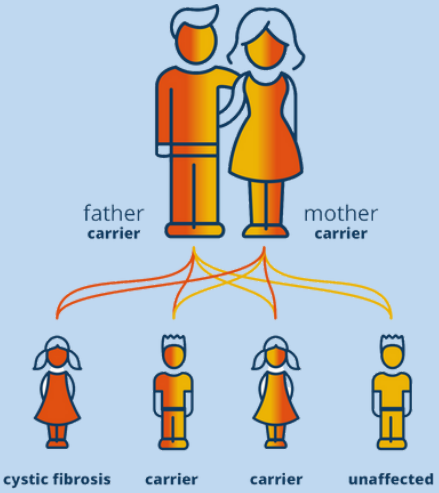
**50%**

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.