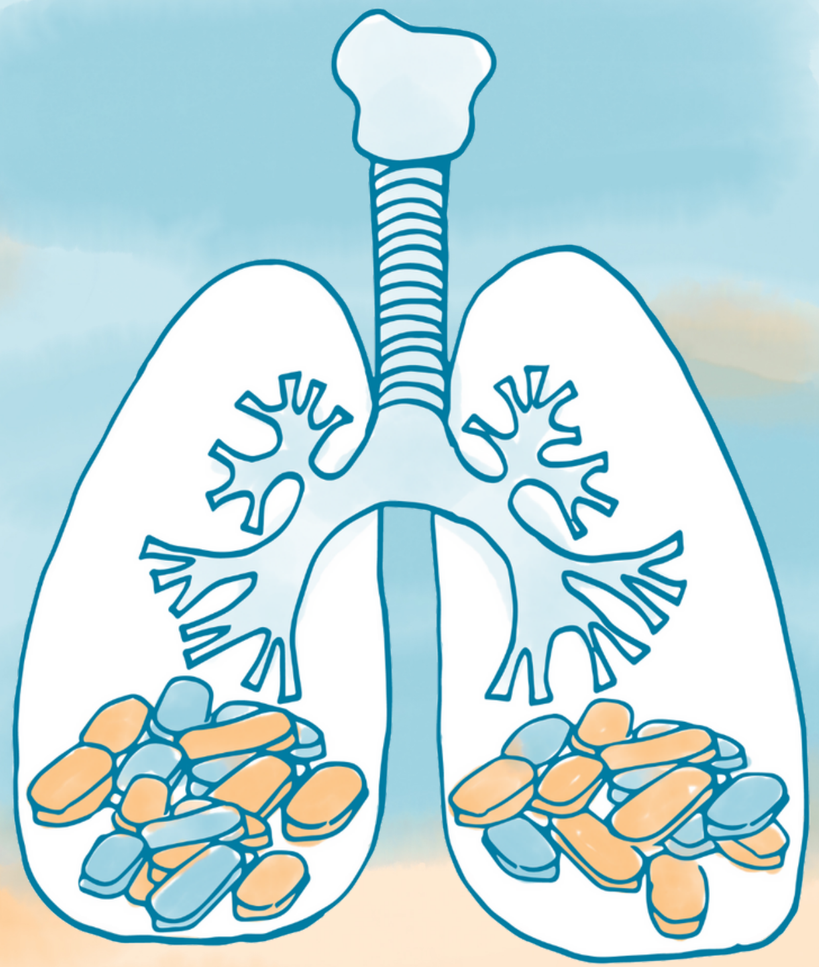


TRIKAFTA - NEXT STEPS

for our CF community

In less than a month, people with cystic fibrosis (CF) - over the age of 6 who meet the eligibility criteria, will have access to funded Trikafta.

From 1 April 2023 New Zealand will now join the more than 30 countries where Trikafta is publicly funded.

CYSTIC FIBROSIS NZ

SUMMARY

In less than a month, people with cystic fibrosis (CF) - over the age of 6 who meet the eligibility criteria, will have access to funded Trikafta. From 1 April 2023 New Zealand will now join the more than 30 countries where Trikafta is publicly funded.

It is important to be aware of the criteria that people with CF are required to meet to be eligible for Trikafta in New Zealand. Please refer to the eligibility criteria below. If you have any questions or if you're unsure about whether you are eligible, please speak with your CF Team.

Pharmac is pleased to be funding a treatment with the potential to greatly improve the health outcomes of people living with CF, and quality of life for families, and whānau.

The feedback they received from the consultation reflected what they had heard from the CF community and clinical advisors when assessing the application for Trikafta.

Pharmac acknowledged that they are grateful to those who took the time to provide feedback to the proposal, in particular those who courageously shared their personal stories.

WAS THERE ANY CHANGE TO THE ORIGINAL PROPOSAL FOR TRIKAFTA?

No changes were made from the original proposal following feedback from submissions received.

A summary of all the responses and feedback is included in Pharmac's Notification document which you can find here:

https://pharmac.govt.nz/news-and-resources/consultations-and-decisions/2023-03-07-decision-to-fund-elexacaftor-with-tezacaftor-and-ivacaftor-trikafta-for-people-with-cystic-fibrosis

TESTS NEEDED BEFORE STARTING TRIKAFTA

There are some steps to go through before your CF Clinician can prescribe Trikafta:

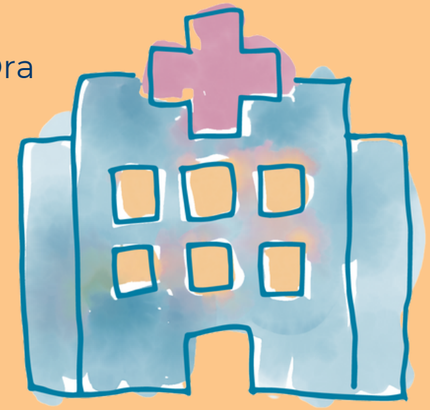
- All patients are recommended to have a blood test to check liver function – your CF team will advise what's needed.
- Under 18s are required to have an eye test to check for cataracts prior to starting on Trikafta for the first time:
  - Eye tests can be done privately if hospital waitlists are too long.
  - Speak to your CF Team to confirm what you need to ask for.
  - If you need support with the cost of paying for a private eye test, please speak to your CFNZ Social Worker.

HOW WILL GETTING ACCESS TO TRIKAFTA WORK?

- All your CF Teams want to get their patients access to Trikafta at the earliest possible opportunity. However, it will not be possible for everyone to start the medicine at the same time.
- Your CF Team will work through their own process to prioritise patients, complete their testing, and provide prescriptions, which means that start dates will be staggered. A staggered start will assist your CF team to safely monitor you as our community starts Trikafta.
- Vertex has confirmed there will be sufficient stocks of Trikafta in New Zealand ahead of 1 April 2023. They have assured us that no one who is eligible for Trikafta will miss out.
- A Special Authority Number, which is needed for the prescription, can only be generated electronically after Trikafta has been loaded on to the Pharmaceutical Schedule.
- Trikafta will be loaded on to the Pharmaceutical Schedule from 1 April 2023.
- Trikafta can only be prescribed from 1 April 2023 (please note, this is a Saturday).
- Patients receiving Trikafta on the Vertex Managed Access Programme will continue to receive the medicine on this programme until they can be commenced on the Pharmac reimbursed medicine by their CF Clinician. Speak to your CF Team if you need more information.

COLLECTING YOUR TRIKAFTA

Trikafta will only be dispensed through Te Whatu Ora inpatient hospital pharmacies, not from Te Whatu Ora outpatient / retail or community pharmacies.



CONTINUING WITH EXISTING MEDICINES AND TREATMENTS

- Clinical advice is that it's important to continue with existing therapies (physio and nebulising) and medications. Please don't stop anything without discussing it with your CF Team.
- Clinicians also advise that exercise will remain an important part of health and wellbeing, don't stop exercise because you're feeling better.

CRITERIA

The approved eligibility criteria for Trikafta are as follows:

Special Authority for Subsidy.
Initial application from any relevant practitioner. Approvals valid without further renewal unless notified for applications meeting the following criteria:

All of the following:

1. Patient has been diagnosed with cystic fibrosis; and
2. Patient is 6 years of age or older; and
3. *Either:*

   3.1. Patient has two cystic fibrosis-causing mutations in the cystic fibrosis transmembrane regulator (CFTR) gene (one from each parental allele); or
   3.2. Patient has a sweat chloride value of at least 60 mmol/L by quantitative pilocarpine iontophoresis or by Macroduct sweat collection system; and

4. *Either*:

   4.1. Patient has a heterozygous or homozygous F508del mutation; or
   4.2. Patient has a G551D mutation or other mutation responsive in vitro to elexacaftor/tezacaftor/ivacaftor (see note a); and
5. The treatment must be the sole funded CFTR modulator therapy for this condition; &
6. Treatment with elexacaftor/tezacaftor/ivacaftor must be given concomitantly with standard therapy for this condition.

Notes:
a) Eligible mutations are listed in the Food and Drug Administration (FDA) Trikafta prescribing information
https://www.accessdata.fda.gov/drugsatfda_docs/label/2021/212273s004lbl.pdf.

RARE MUTATIONS

Rare mutations are reviewed on a regular basis. If additional rare mutations get added to the FDA list, anyone with these mutations who meet the access criteria would also be eligible to receive Trikafta.

Some people may have rare mutations which evidence shows respond to Trikafta, but are not covered by the access criteria. Prescribers can apply for a Named Patient Pharmaceutical Assessment (NPPA) for people with these rare mutations.

You should speak to your prescriber about these options.

UPDATE ABOUT WIDENING OF ACCESS TO KALYDECO AND FUNDING OF SYMDEKO

In CFNZ 's submission to Pharmac and in the community's submission template, we highlighted the need to widen access to Kalydeco as part of the final agreement, and requested that funding of Symdeko be considered.

Unfortunately, neither medicine has been included in the final agreement between Pharmac and Vertex. This wasn't the news we were hoping for. Below is the response from Pharmac:

- *A proposal for widened access to ivacaftor (Kalydeco) is currently under assessment, and will then be ranked on our Options for Investment list (application tracker can be viewed here).*
- *We would welcome a funding application for tezacaftor with ivacaftor (Symdeko). We have shared this feedback with the supplier, Vertex, to highlight the public support for a funding application for Symdeko in New Zealand.*
- *We acknowledge the strong desire for a funded CFTR treatment that will work for those with cystic fibrosis who are eligible. This is something we are committed to working on with patient advocate groups, with Vertex, and the wider community.*

CFNZ has urged Pharmac to widen access to Kalydeco and provide access to other CFTR therapies to everyone who would benefit, as quickly as practically possible. We have highlighted that there is no equity until everyone who can benefit from a CFTR therapy, has one.

CFNZ will continue to advocate for this to happen and fight for fair access for everyone who would benefit.