Provincial Health Services Authority

Frequently Asked Questions about Trikafta®

A large number of patients with cystic fibrosis (CF) in British Columbia may qualify for a prescription drug called Trikafta®, which has now been approved by Health Canada for ages 2+ (since October 2023). There are a number of private insurers which cover Trikafta® for patients who have extended health coverage, and the Ministry of Health announced BC PharmaCare would provide exceptional case by case coverage of Trikafta® through the BC Expensive Drugs for Rare Diseases (EDRD) process. Your CF care team has compiled this list of frequently asked questions about Trikafta®. If you have further questions after reading this document, please feel free to reach us at **cfcarebc@phsa.ca**.

WHAT IS TRIKAFTA®?

Trikafta® is a prescription drug therapy approved by Health Canada for patients 2 years of age or older with cystic fibrosis who have at least one F508del gene variant in the cystic fibrosis transmembrane conductance regulator (CFTR) gene. Trikafta® is also known as triple-combination therapy, a medication made up of three different modulators: tezacaftor/ivacaftor combined with elexacaftor.

What is CFTR?

The CFTR protein helps to maintain the balance of salt and water on many surfaces of the body, including the lungs. The main function of CFTR in the body is to regulate the water content of mucus. When there is a defect in the CFTR protein, the mucus is thick and sticky and can cause many of the symptoms associated with CF.

The instructions on how to make the CFTR protein are contained in the CFTR gene, which is a small part of everyone's DNA. Different people have many different variants in their CFTR gene that can result in a CFTR protein that does not function properly.

This 40-second video [1] from the CF Foundation shows what it looks like when the CFTR protein functions normally in the lungs, and when it doesn't.

NOTE: the video has no sound.

What are CFTR modulator drugs?

CFTR modulators are a new type of medication that work directly on the dysfunctional CFTR protein to help repair the underlying cause of the excessively thick mucus. The type of CFTR modulator medication must be matched to the specific CFTR gene variants seen in an individual with CF.

Would Trikafta® work for my CFTR variants?

You can ask your CF physician at your next CF clinic visit which CFTR variants you have. If you have one copy of F508del you may be eligible for Trikafta®.

How will Trikafta® help my CF?

The clinical studies of Trikafta® showed improvements in measurements of lung function, rate of lung infection (e.g. pulmonary exacerbations), growth, and symptoms. The degree of benefit on average is greater than the improvements seen with other CF medications, but individual responses can vary.

GETTING ACCESS: INSURANCE AND ENROLLMENT

What is the eligibility criteria for Trikafta®?

Trikafta® drug therapy is approved for people 2 years and older with CF who have at least one F508del variant in the CF transmembrane conductance regulator (CFTR) gene.

How much does Trikafta® cost?

Trikafta® is priced around $300,000 per person per year.

What are the steps to determine who can get Trikafta®?

After a new drug is approved by Health Canada, there are different ways that the medication can be accessed:

1. Covered under private (third-party) insurance plans
2. Covered under the province on a case-by-case basis through the EDRD review process
3. Directly paid for by the individual

Will private insurance cover Trikafta®?

Some private insurance plans will cover Trikafta®. Most require a prior authorization form to be submitted, and will reach a decision on coverage after a review process. This process may take up to 3 months, but can be longer.

If Trikafta® coverage is approved, then details of coverage including percentage of cost covered, deductible amounts, and annual or lifetime maximums will be determined.

What are the next steps if I have private insurance?

Check if your insurance plan provider lists Trikafta® for coverage:**Weight and Age Group**	**Trikafta® Strength**	**DIN number**
2-5 years old, less than 14 kg	Trikafta® 80/40/60 + 59.5 mg granules	02542285
2-5 years old, more than 14 kg	Trikafta® 100/50/75 + 75 mg granules	02542277
6-11 years old, less than 30 kg	Trikafta® 50/25/37.5 + 75 mg tablets	02526670
6 years and older, more than 30 kg	Trikafta® 100/50/75 + 150 mg tablets	02517140

If listed for coverage or if prior authorization forms are required, then we would recommend that you enroll into the Village™ Patient Support Program. This is a patient support program set up by Vertex, that helps patients and families navigate insurance for coverage. The enrollment process will begin with a discussion with your CF team at your next clinic visit or by phone. You will complete an enrollment form and give your signed consent for sharing your contact information, insurance status, and personal health data with the program including for commercial purposes.

Enrollment into the Village™ Patient Support Program is not required for access to Trikafta®.

If not listed for coverage or if coverage is denied, then please ask your drug insurance plan to send you a letter of denial stating that Trikafta coverage is denied. This letter of denial will be required for application to the province (EDRD) for coverage.

What are the next steps if I do not have private insurance?

The CF Clinics are using their clinic data and the Canadian CF Registry to identify all people with CF that have at least one copy of the F508del variant, and would be potentially eligible for Trikafta® therapy. They will arrange your baseline investigations and complete the paperwork required by the (EDRD) program to approve provincial coverage.

How will the CF clinics prioritize the order in which people get access to Trikafta®?

Many factors will be considered prior to starting eligible people with CF on Trikafta® and there is a province-wide plan to ensure timely access to the medication. Some considerations may include medical condition at time of eligibility, third-party drug coverage application processes, and people with CF on compassionate access programs. Your CF clinics will be working hard to roll out Trikafta® in an organized manner. As of 2024, many people have already started Trikafta®, so as younger age groups are eligible, your CF Clinic will contact you to get the process started.

TRIKAFTA TREATMENT: RESULTS AND INTERACTIONS

What do I need to get started on Trikafta®?

Before you start on Trikafta®, there are required tests to be completed. This is to determine your baseline measurements and may include sweat chloride, lung function, blood tests of liver function, degree of lung disease (chest CT scan), mental health measures and symptom burden. Your CF clinic will contact you to book these tests.

Follow-up testing will be done after starting on Trikafta® to assess both safety and beneficial drug effects. This follow-up testing is necessary in order to maintain coverage of Trikafta®.

What are common side effects of Trikafta®?

Common adverse effects may include abdominal pain, rash, headache, diarrhea, nasal congestion and sinus symptoms and abnormalities in blood tests of liver function blood and muscle enzymes. Your prescriber and CF team will be monitoring for drug-related side effects and safety. Please consult with your prescriber regarding individual questions about adverse effects.

Does Trikafta® interact with other medications used by people with CF?

Certain drugs may interact with Trikafta®, including some antifungal medications (e.g. itraconazole, voriconazole), some antibiotics (e.g. rifampin and rifabutin), some seizure medications (e.g. phenobarbital, carbamazepine, phenytoin), and some natural or herbal medications (St. John's Wort). Grapefruit, pomelo and seville oranges, can also interact with Trikafta®, so food and drinks containing this ingredient should be avoided while on Trikafta®.

How will my response to Trikafta® be monitored?

Follow-up testing may include sweat chloride, lung function, blood tests of liver function, chest CT scan, stool tests, mental health measures, and measurement of quality of life scores will be done after being on Trikafta® to monitor drug effects. This is for both drug safety and effectiveness. Compliance to follow up monitoring is required to maintain Trikafta® coverage and access.

Can I stop using my other CF medications once I start on Trikafta®?

Please do not stop any of your therapies without speaking to your CF team. The studies of Trikafta® were done in people with CF who were also taking all their usual CF medications (e.g. enzymes, Pulmozyme®, hypertonic saline, inhaled antibiotics), so we know that the addition of Trikafta® to usual therapy results in the previously mentioned improvements in lung function and other outcomes.

We do not yet know if any CF therapies can be safely stopped once someone is on Trikafta®. There are studies underway to see if certain CF medications can be safely stopped without a negative effect on health.

CONDITIONS PREVENTING USE OF TRIKAFTA®

What medical conditions would prevent the use of Trikafta®?

Trikafta® should not be used in people with CF who have severe liver disease. People with CF who have liver disease must be monitored very closely with bloodwork, clinical assessment and other tests (e.g. ultrasound) if they are on Trikafta®. Your CF doctor can determine if it is safe for you to use Trikafta®.

Can I use Trikafta® if I am pregnant or am trying to get pregnant?

Trikafta® may increase fertility in women with CF due to its impact on the mucus in the cervix and uterus. The studies of Trikafta® were not done in women with CF who were pregnant, so we do not know the effect of this drug on a developing fetus. Please talk to your prescriber or CF team before considering pregnancy to get up-to-date information.

Can I use Trikafta® if I have had a lung transplant?

Trikafta® will not have an impact on lung function after a lung transplant. When you have received a lung transplant, your new lungs do not have CF or the defect in the CFTR protein. Trikafta® has not been tested in people with CF who have had a lung transplant, so we do not know if it would help other aspects of CF such as nutrition or sinus symptoms. Trikafta® can interact with immunosuppressive drugs needed after transplant, therefore the decision to use Trikafta® with a lung transplant should be discussed with your transplant team.

Can I use Trikafta® if I have had a liver transplant?

Trikafta® can be used in people who have had a liver transplant to help improve lung function. However, due to interactions between Trikafta® and some immunosuppressive medications, discuss how you will monitor this with your transplant team before starting Trikafta®.

HOW CAN I GET MORE INFORMATION ABOUT TRIKAFTA®?

- CADTH Reimbursement Recommendations published in the Canadian Journal of Health Technologies
  - December 2023
  - July 2022
  - September 2021
- Canadian Clinical Consensus Guideline for Initiation, Monitoring and Discontinuation of CFTR Modulator Therapies for Patients with Cystic Fibrosis
- Pan Canadian Pharmaceutical Alliance
- BC Ministry of Health Drug Review Process
- Cystic Fibrosis Canada – Trikafta®

[1] https://www.cff.org/Life-With-CF/Treatments-and-Therapies/Medications/CFTR-Modulator-Therapies/