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 received a notice of compliance from Health Canada in June 2021. 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 on October 5, 2021. 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 6 years of age or older with cystic fibrosis who have at least one F508del mutation 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 (which make up Symdeko®) combined with elexacaftor. The drug only acts on the CFTR protein, and not on the genetic material in the body.

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 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.

How do I find out about my CFTR variants?

1. You can ask your CF physician at your next CF clinic visit.
2. Alternatively, you can look up your CF mutation on the CF Canada Patient Registry. You can register to have access to this patient database at your CF clinic visit or by contacting cfregistry@cysticfibrosis.ca
3. If you have provided email consent, your local CF clinic can email you the names of your CF mutations and whether you are eligible for Trikafta®.

GETTING ACCESS: INSURANCE AND ENROLLMENT

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 provincial drug plans on a case by case basis through the EDRD review process
3. Directly paid for by the individual

How much does Trikafta® cost?

Trikafta® is priced at $306,800 per year.

Will private insurance cover Trikafta®?

Private insurers perform their own internal review of new medications to decide if they will add them to their list of medications that they will cover. This process takes about one to three months, but can be longer.

Not all insurance plans will cover CFTR modulators, and some will have an annual or lifetime maximum amount of money they will pay for drug coverage. Your private insurer will have information about what medications are covered and if there are maximum limits. Private insurance coverage must be explored and utilized before provincial drug coverage review.

What are next steps if I have private insurance?

Contact your insurance plan provider and ask if they cover Trikafta® (Drug Identification Number DIN 02517140).

If covered, also ask your insurance plan provider for a letter stating it is covered and if there are any annual or lifetime coverage maximums, what they are, and how much you have left of the coverage.

Whether the response is approval or denial of coverage, request the insurance provider to provide you with a letter regarding the decision on Trikafta® coverage. This letter will serve as important documentation when your CF team applies for provincial coverage.

If you and your CF team are unable to secure a letter from your health insurance provider, you could alternatively decide to enroll in the Village™ Patient Support Program, set up by Vertex, the manufacturer of Trikafta®. 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. The Village™ Patient Support Program can assist with securing a letter from your insurance provider.

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

What happens to people who are receiving Trikafta® through the Health Canada Special Access Program and Vertex Compassionate Use Program?

Now that Health Canada has approved Trikafta®, Special Access Program approval is no longer required and everyone in the Vertex Compassionate Use Program must enroll in the Village™ Patient Support Program. Coverage through the Vertex Compassionate Use Program will continue indefinitely until Trikafta® is available either through the individual's private insurance or through the provincial drug plan.

What are the CF Clinics doing to help eligible people with CF get access to Trikafta®?

- The CF Clinics are using the Canadian CF Registry to identify all people with CF that have at least one copy of the deltaF508 mutation, and would be potentially eligible for Trikafta® therapy.
- They will also receive direct updates about which insurance plans have agreed to cover Trikafta®.
- They will be completing the paperwork needed by private insurers (third-party payers) and the Expensive Drugs for Rare Diseases (EDRD) program to approve coverage.
- They will aim to increase the number of sweat testing facilities in the province to improve capacity and ease of access to testing.

How will the CF clinics prioritize the order in which people are enrolled?

Many factors will be considered prior to starting eligible people with CF on Trikafta® and there will be 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. If your child is eligible, your CF clinic will be in contact with you.

What is the eligibility criteria?

Trikafta® drug therapy is approved for people 6 years and older with CF who have at least one F508del mutation in the CF transmembrane conductance regulator (CFTR) gene; however, it is still in the process of being approved for public insurance coverage.

Will there be a FEV1% of 90% ceiling?

The restriction for eligibility of Trikafta® for those with FEV1% of over 90% appears to have been removed. This is still under review through the provincial review process and updates will be provided as soon as possible.

Will my private insurance company cover Trikafta®?

Contact your private insurance plan provider and ask if they cover Trikafta®:

- For people aged 6 and older **AND** weigh 30 kg (65 pounds) or greater: Trikafta® DIN 2517140
- For people aged 6 and older **AND** weigh less than 30 kg (65 pounds): Trikafta® DIN 2526670

If your insurance plan covers Trikafta®, please ask your insurance plan provider for a letter stating:

- Your insurance plan is covered
- What the annual or lifetime coverage maximums are (if any)
- How much you have left of the coverage

Whether the response is approval or denial of coverage, request the insurance provider to provide you with a letter regarding the decision on Trikafta® coverage. This letter will serve as important documentation when your CF team applies for provincial coverage.

Are there any baseline investigations we should do now?

Your CF clinic will be in contact with you to arrange required baseline investigations as part of the Trikafta® enrollment process requirements. At the moment there are no actions required: Your CF clinic will contact you when it is time to arrange any testing.

Access more information

CADTH & Trikafta review

Pan Canadian Pharmaceutical Alliance

BC Ministry of Health Drug Review Process

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 of sweat chloride, lung function, blood tests of liver function, degree of lung disease (chest CT scan), and symptom burden. Consult your CF clinic about scheduling 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®.

How will Trikafta® help my CF?

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

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®.

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 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 including sweat chloride, lung function, blood tests of liver function, chest CT scan 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.

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 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. 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 is a study underway in the United States to see if certain CF medications can be safely stopped without a negative effect on health.

HOW CAN I GET MORE INFORMATION ABOUT TRIKAFTA®?

Read the **CADTH Reimbursement Recommendation** [2] in the September 2021 issue of the **Canadian Journal of Health Technologies** [3].

Canadian Clinical Consensus Guideline for Initiation, Monitoring and Discontinuation of CFTR Modulator Therapies for Patients with Cystic Fibrosis [4]

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

[2] https://cadth.ca/sites/default/files/DRR/2021/SR0673%20Trikafta%20%20CADTH%20Final%20Rec%20Revised.pdf

[3] https://canjhealthtechnol.ca/index.php/cjht/issue/view/10

[4] https://www.cysticfibrosis.ca/uploads/CFC%20Modulator%20Guidelines_RevisedOct62021%20(003).pdf