BC PharmaCare Drug Information

The drug below is being considered for coverage under the BC PharmaCare program. PharmaCare is a government-funded drug plan that helps B.C. residents with the cost of eligible prescription drugs and medical supplies. For more information about PharmaCare, visit the PharmaCare website.

PharmaCare reviews each drug for treating a specific illness or medical condition (also called an "indication"). If PharmaCare decides to cover a drug, that coverage applies only to the indication(s) specified. In some cases, PharmaCare covers a drug only for people who have not responded to other drugs that treat the same indication.

More information about the PharmaCare drug review process is provided on the last page of this document.

Drug information	
Generic name (scientific name)	**Elexacaftor/tezacaftor/ivacaftor and ivacaftor**
Brand name	**Trikafta®**
Manufacturer	Vertex Pharmaceuticals (Canada) Incorporated
Indication	For the treatment of cystic fibrosis in children ages 2+
Has the drug been reviewed by CDA-AMC, or will CDA-AMC be reviewing it? (See note below.)	Yes For more information about the CDA-AMC Reimbursement Review (CRR) of elexacaftor/tezacaftor/ivacaftor and ivacaftor, Search the CDA-AMC Reports.
Public input start date	Wednesday, June 26, 2024
Public input closing date	**Tuesday, July 23, 2024 at 11:59 PM**
How is the drug taken?	Trikafta is taken orally (by the mouth).
How often is the drug taken?	Trikafta is taken once in the morning and once in the evening. Doses should be spaced 12 hours apart.

General drug and/or drug study information	Elexacaftor/tezacaftor/ivacaftor and ivacaftor is being reviewed for the treatment of cystic fibrosis (CF) in patients aged 2 years and older who have at least one F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene or a mutation in the CFTR gene that is responsive based on in vitro[a] and/or clinical data. CF is an inherited disease that causes thick, sticky mucus to build up in the lungs and digestive tract. Symptoms can include a persistent cough, wheezing, inability to exercise, lung infections, sinus inflammation and infections, poor weight gain and growth, and severe constipation. People with CF are at risk for complications such as diabetes, liver disease, and nutritional deficiencies. CF is caused by mutations in the CF gene. These mutations lead to defects in a specific protein called the cystic fibrosis transmembrane conductance regulator (CFTR) protein. As a result of these defects, the CFTR proteins don't work the way they should. Elexacaftor and tezacaftor are both CFTR correctors, which means they fix the defective proteins, allowing more of the proteins to move to their proper position on the cell surface. Ivacaftor, which is known as a potentiator, then allows CFTR proteins on the surface to stay open longer, which keeps important pathways open. Studies looked at the following: • Absolute change from baseline in percent predicted forced expiratory volume in 1 second ($ppFEV_1$) through week 24 • Absolute change from baseline in sweat chloride (SwCl) through week 24 • Absolute change from baseline in cystic fibrosis questionnaire-revised (CFQ-R RD) score through week 24 • Absolute change from baseline in body mass index (BMI)[b] and BMI z-score[c] at week 24. • Absolute change from baseline in body weight (kg) and body weight z-score[c] at week 24 • Number of pulmonary exacerbations (PEx) through week 24 • Bad reactions • Serious bad reactions • Patients leaving the trial due to bad reactions

Drug information	
	• Bad reactions of special interest, such as elevated liver enzymes and rash
Other considerations	None

Note:
CDA-AMC (Canada's Drug Agency-L'Agence des Médicaments du Canada) is a national organization that reviews drugs on behalf of Canadian public sector plans when drug manufacturers want those plans to provide coverage for the drug. For detailed information about the PharmaCare drug review process, including the role of the CDA-AMC Reimbursement Review (CRR) in that process, visit How PharmaCare Decides Which Drugs to Cover.

Cost of the drug compared to other drugs used to treat the same indication				
Generic Name (Brand Name) of Drug Comparator	**PharmaCare Status (if and how the drug is already covered)**	**Dosage Form**	**Usual Dose**	**Annual Cost of Therapy**
elexacaftor/tezacaftor/ivacaftor and ivacaftor (Trikafta)	Under Review	Tablet, Granules Packet	Twice daily, 12 hours apart	$307,000
CFTR modulator therapies				
ivakaftor (Kalydeco)	Exceptional, case-by-case coverage provided through the BC EDRD program	Granules Packet	Twice daily, 12 hours apart	$307,000

[a] In vitro means happening outside the body in artificial conditions, often in a test tube.
[b] BMI is a person's weight in kilograms (or pounds) divided by the square of height in meters (or feet).
[c] A z-score is a tool that shows how a patient's measurements (height, weight, BMI) compare to other people of the same age and sex. A positive z-score means the measurement is higher than the average, while a negative z-score means it is lower than average.
[d] All prices as per PharmaCare Formulary, unless otherwise indicated.

The Drug Review Process in B.C.

A manufacturer submits a request to the Ministry of Health (Ministry).

An independent group called the Drug Benefit Council (DBC) gives advice to the Ministry. The DBC looks at:

- whether the drug is safe and effective
- advice from a national group called Canada's Drug Agency-L'Agence des médicaments du Canada (CDA-AMC)
- what the drug costs and whether it is a good value to the citizens of B.C.
- ethical considerations related to covering or not covering the drug
- input from physicians, patients, caregivers, patient groups and drug submission sponsors

The Ministry makes PharmaCare coverage decisions by taking into account:

- existing PharmaCare policies, programs and resources
- the evidence-informed advice of the DBC
- the drugs already covered by PharmaCare to treat similar medical conditions
- the overall cost of covering the drug

For more information about the drug review process in B.C., visit: the How PharmaCare decides which drugs to cover.

This document provides information only.
It does not take the place of advice from a physician or other qualified health care provider.