PATIENT & CAREGIVER EDUCATION

Lumacaftor and Ivacaftor

This information from UpToDate® Lexidrug™ explains what you need to know about this medication, including what it's used for, how to take it, its side effects, and when to call your healthcare provider.

Brand Names: US

Orkambi

Brand Names: Canada

Orkambi

What is this drug used for?

- It is used to treat cystic fibrosis.

What do I need to tell my doctor BEFORE I take this drug?

- If you are allergic to this drug; any part of this drug; or any other drugs, foods, or substances. Tell your doctor about the allergy and what signs you had.
- If you have had an organ transplant.
- If you are taking any of these drugs: Carbamazepine, phenobarbital, phenytoin, rifabutin, rifampin, or St.

John's wort.

- If you are taking any of these drugs: Cyclosporine, everolimus, midazolam, sirolimus, tacrolimus, or triazolam.
- If you are taking birth control pills or some other hormone-based birth control.

This is not a list of all drugs or health problems that interact with this drug.

Tell your doctor and pharmacist about all of your drugs (prescription or OTC, natural products, vitamins) and health problems. You must check to make sure that it is safe for you to take this drug with all of your drugs and health problems. Do not start, stop, or change the dose of any drug without checking with your doctor.

What are some things I need to know or do while I take this drug?

- Tell all of your health care providers that you take this drug. This includes your doctors, nurses, pharmacists, and dentists.
- Have your blood work and lung function checked as you have been told by your doctor.
- High blood pressure has happened with this drug. Have your blood pressure checked as you have been told by

your doctor.

- Cataracts have happened in children taking this drug. Have an eye exam before starting this drug and while taking it. Talk with your doctor.
- Liver problems have happened with this drug. Liver problems have also gotten worse in people who already have them. Rarely, this has been deadly. If you have questions, talk with the doctor.
- Birth control pills and other hormone-based birth control may not work as well to prevent pregnancy. Use some other kind of birth control also like a condom when taking this drug.
- Tell your doctor if you are pregnant, plan on getting pregnant, or are breast-feeding. You will need to talk about the benefits and risks to you and the baby.

What are some side effects that I need to call my doctor about right away?

WARNING/CAUTION: Even though it may be rare, some people may have very bad and sometimes deadly side effects when taking a drug. Tell your doctor or get medical help right away if you have any of the following signs or symptoms that may be related to a very bad side effect:

- Signs of an allergic reaction, like rash; hives; itching; red, swollen, blistered, or peeling skin with or without fever;

wheezing; tightness in the chest or throat; trouble breathing, swallowing, or talking; unusual hoarseness; or swelling of the mouth, face, lips, tongue, or throat.

- Signs of liver problems like dark urine, tiredness, decreased appetite, upset stomach or stomach pain, light-colored stools, throwing up, or yellow skin or eyes.
- Signs of high blood pressure like very bad headache or dizziness, passing out, or change in eyesight.
- Signs of new or worsening mental or mood problems like anxiety, depression, suicidal thoughts or actions, or trouble sleeping.
- Change in eyesight.
- Chest pain or pressure.
- Shortness of breath.
- Trouble breathing that is new or worse.
- Feeling confused.
- Raised pressure in the brain has happened during treatment with this drug and other drugs like it. The risk may be raised in people with high vitamin A levels. Call your doctor right away if you have a headache; dizziness; upset stomach; ringing in the ears; eye pain; or eyesight problems like blurred eyesight, seeing double, or loss of eyesight.

What are some other side effects of this drug?

All drugs may cause side effects. However, many people have no side effects or only have minor side effects. Call your doctor or get medical help if any of these side effects or any other side effects bother you or do not go away:

- Stomach pain or diarrhea.
- Upset stomach.
- Feeling tired or weak.
- Gas.
- Flu-like signs.
- Signs of a common cold.
- Nose or throat irritation.
- Cough.
- More sputum.
- Headache.
- Period (menstrual) changes.

These are not all of the side effects that may occur. If you have questions about side effects, call your doctor. Call your doctor for medical advice about side effects.

You may report side effects to your national health agency.

You may report side effects to the FDA at 1- 800-332-1088. You may also report side effects at https://www.fda.gov/medwatch.

How is this drug best taken?

Use this drug as ordered by your doctor. Read all information given to you. Follow all instructions closely.

All products:

- Take this drug just before or just after fatty foods. There are many fatty foods. Some of these are eggs; butter; peanut butter; cheese pizza; whole-milk dairy products like whole-milk, cheese, or yogurt; breast milk; or infant formula. If you are not sure what types of foods are fatty, talk with your doctor.
- Keep taking this drug as you have been told by your doctor or other health care provider, even if you feel well.
- Do not eat grapefruit or drink grapefruit juice during the first week you are taking this drug.

Granules:

- Before you open the packet, shake gently so the granules move to the bottom of the packet.
- Mix the contents of the packet with 1 teaspoon (5 mL) of soft food or liquid. This includes pureed fruits or

vegetables, flavored yogurt or pudding, applesauce, water, breast milk, baby formula, milk, or juice. Food or liquid needs to be at or below room temperature.

- Take this drug within 1 hour of mixing.

What do I do if I miss a dose?

- Take a missed dose as soon as you think about it.
- If it has been 6 hours or more since the missed dose, skip the missed dose and go back to your normal time.
- Do not take 2 doses at the same time or extra doses.
- If you miss 7 days of this drug, call your doctor to find out what to do.

How do I store and/or throw out this drug?

- Store at room temperature in a dry place. Do not store in a bathroom.
- Keep all drugs in a safe place. Keep all drugs out of the reach of children and pets.
- Throw away unused or expired drugs. Do not flush down a toilet or pour down a drain unless you are told to do so. Check with your pharmacist if you have questions about the best way to throw out drugs. There may be drug take-back programs in your area.

General drug facts

- If your symptoms or health problems do not get better or if they become worse, call your doctor.
- Do not share your drugs with others and do not take anyone else's drugs.
- Some drugs may have another patient information leaflet. If you have any questions about this drug, please talk with your doctor, nurse, pharmacist, or other health care provider.
- Some drugs may have another patient information leaflet. Check with your pharmacist. If you have any questions about this drug, please talk with your doctor, nurse, pharmacist, or other health care provider.
- If you think there has been an overdose, call your poison control center or get medical care right away. Be ready to tell or show what was taken, how much, and when it happened.

Consumer Information Use and Disclaimer

This generalized information is a limited summary of diagnosis, treatment, and/or medication information. It is not meant to be comprehensive and should be used as a tool to help the user understand and/or assess potential diagnostic and treatment options. It does NOT include all information about conditions, treatments, medications,

side effects, or risks that may apply to a specific patient. It is not intended to be medical advice or a substitute for the medical advice, diagnosis, or treatment of a health care provider based on the health care provider's examination and assessment of a patient's specific and unique circumstances. Patients must speak with a health care provider for complete information about their health, medical questions, and treatment options, including any risks or benefits regarding use of medications. This information does not endorse any treatments or medications as safe, effective, or approved for treating a specific patient. UpToDate, Inc. and its affiliates disclaim any warranty or liability relating to this information or the use thereof. The use of this information is governed by the Terms of Use, available at https://www.wolterskluwer.com/en/know/clinical-effectiveness-terms.

Last Reviewed Date

2026-04-14

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If you have questions or concerns, contact your healthcare provider. A member of your care team will answer Monday through Friday from 9 a.m. to 5 p.m. Outside those hours, you can leave a message or talk with another MSK provider. There is always a doctor or nurse on call. If you're not sure how to reach your healthcare provider, call 212-639-2000.

For more resources, visit www.mskcc.org/pe to search our virtual library.

Lumacaftor and Ivacaftor - Last updated on December 12, 2022
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