

# EVERY DOSE MATTERS WITH ORKAMBI<sup>®</sup>

A guide for helping your child take ORKAMBI



## What is ORKAMBI<sup>®</sup> (lumacaftor/ivacaftor)?

ORKAMBI is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients age 2 years and older who have two copies of the F508del mutation (F508del/F508del) in their CFTR gene.

ORKAMBI should not be used in patients other than those who have two copies of the F508del mutation in their CFTR gene.

It is not known if ORKAMBI is safe and effective in children under 2 years of age.

**Please see [Important Safety Information](#) on pages 2-4 and full [Prescribing Information](#), including [Patient Information](#).**

Photos in this brochure are of people with CF who have 2 copies of the F508del-CFTR mutation or their caregivers. The people with CF may or may not be taking ORKAMBI.

## IMPORTANT SAFETY INFORMATION

### Your child should not take ORKAMBI<sup>®</sup> if he or she takes certain medicines or herbal supplements, such as:

- antibiotics: rifampin (Rifamate<sup>®</sup>, Rifater<sup>®</sup>) or rifabutin (Mycobutin<sup>®</sup>)
- seizure medicines: phenobarbital, carbamazepine (Tegretol<sup>®</sup>, Carbatrol<sup>®</sup>, and Equetro<sup>®</sup>), or phenytoin (Dilantin<sup>®</sup>, Phenytek<sup>®</sup>)
- sedatives and anti-anxiety medicines: triazolam (Halcion<sup>®</sup>) or midazolam (Dormicum<sup>®</sup>, Hypnovel<sup>®</sup>, and Versed<sup>®</sup>)
- immunosuppressant medicines: cyclosporine, everolimus (Zortress<sup>®</sup>), sirolimus (Rapamune<sup>®</sup>), or tacrolimus (Astagraf XL<sup>®</sup>, Envarsus XR<sup>®</sup>, Prograf<sup>®</sup>, and Protopic<sup>®</sup>)
- St. John's wort (*Hypericum perforatum*)

Talk to your child's doctor before taking ORKAMBI if he or she takes any of the medicines or supplements listed above.

### Before taking ORKAMBI, tell your child's doctor about all of your child's medical conditions, including if he or she:

- has or has had liver problems
- has had an organ transplant
- has kidney problems
- is using birth control (hormonal contraceptives, including oral, injectable, transdermal, or implantable forms). Hormonal contraceptives should not be used as a method of birth control when taking ORKAMBI. Talk to the doctor about the best birth control method to use while taking ORKAMBI
- is pregnant or plans to become pregnant. It is not known if ORKAMBI will harm an unborn baby. Patients and their doctor should decide whether ORKAMBI should be taken during pregnancy
- is breastfeeding or planning to breastfeed. It is not known if ORKAMBI passes into breast milk. Patients and their doctor should decide whether ORKAMBI should be taken while breastfeeding

### ORKAMBI<sup>®</sup> (lumacaftor/ivacaftor) may affect the way other medicines work, and other medicines may affect how ORKAMBI works.

Tell your child's doctor about all the medicines he or she takes, including prescription and over-the-counter medicines, vitamins, and herbal supplements, because the dose of ORKAMBI may need to be adjusted when taken with certain medicines.

Especially tell your child's doctor if he or she takes:

- antifungal medicines including ketoconazole (such as Nizoral<sup>®</sup>), itraconazole (such as Sporanox<sup>®</sup>), posaconazole (such as Noxafil<sup>®</sup>), or voriconazole (such as Vfend<sup>®</sup>)
- antibiotics including telithromycin (such as Ketek<sup>®</sup>), clarithromycin (such as Biaxin<sup>®</sup>), or erythromycin (such as Ery-Tab<sup>®</sup>)

### Tell your child's doctor if he or she stops ORKAMBI for more than 1 week.

Your child's doctor may need to change the dose of ORKAMBI or other medicines your child takes.

### What are the possible side effects of ORKAMBI?

#### ORKAMBI can cause serious side effects, including:

- **Worsening of liver function** in people with severe liver disease. The worsening of liver function can be serious or cause death. Talk to your child's doctor if you have been told that he or she has liver disease as your child's doctor may need to adjust the dose of ORKAMBI
- **High liver enzymes in the blood**, which can be a sign of liver injury in people receiving ORKAMBI. Your child's doctor will do blood tests to check your child's liver:
  - before starting ORKAMBI
  - every 3 months during the first year of taking ORKAMBI
  - every year while taking ORKAMBI

Call your child's doctor right away if he or she has any of the following symptoms of liver problems:

- pain or discomfort in the upper right stomach (abdominal) area
- loss of appetite
- yellowing of the skin or the white part of the eyes
- nausea or vomiting
- dark, amber-colored urine
- confusion

Please see additional [Important Safety Information on page 4](#) and full [Prescribing Information, including Patient Information.](#)

## IMPORTANT SAFETY INFORMATION (CONTINUED)

### ORKAMBI<sup>®</sup> can cause serious side effects, including (continued):

- **Breathing problems** such as shortness of breath or chest tightness in patients when starting ORKAMBI, especially in patients who have poor lung function. If your child has poor lung function, your child's doctor may monitor him or her more closely when starting ORKAMBI
- **An increase in blood pressure** in some people receiving ORKAMBI. Your child's doctor should monitor your child's blood pressure during treatment with ORKAMBI
- **Abnormality of the eye lens (cataract)** in some children and adolescents receiving ORKAMBI. Your child's doctor should perform eye examinations before and during treatment with ORKAMBI to look for cataracts

### The most common side effects of ORKAMBI include:

- breathing problems such as shortness of breath and chest tightness
- nausea
- diarrhea
- fatigue
- increase in a certain blood enzyme called creatine phosphokinase
- rash
- gas
- common cold, including sore throat, stuffy or runny nose
- flu or flu-like symptoms
- irregular, missed, or abnormal periods (menses) and increase in the amount of menstrual bleeding

**Side effects seen in children** are similar to those seen in adults and adolescents. Additional common side effects seen in children include:

- cough with sputum
- stuffy nose
- headache
- stomach pain
- increase in sputum

These are not all the possible side effects of ORKAMBI. Call your child's doctor for medical advice about side effects. You are encouraged to report side effects to FDA at 1-800-FDA-1088.

## How your child should take ORKAMBI<sup>®</sup> (lumacaftor/ivacaftor)

The recommended dose of ORKAMBI is **2 tablets every 12 hours with food that contains fat**. Every dose matters, so it's important that your child take ORKAMBI exactly as prescribed.



Taking ORKAMBI every 12 hours helps keep enough medicine in the body.



Remember, having your child take ORKAMBI every 12 hours is not the same as having him or her take it twice a day.



**Your child should take ORKAMBI, plus all other CF therapies, exactly as your child's healthcare provider says they should be taken.**



Your child's dose may be different. Your child's healthcare provider will tell you how much ORKAMBI your child should take and when it should be taken.

## How ORKAMBI<sup>®</sup> is packaged

Each box of ORKAMBI has 4 weekly cartons. Inside each weekly carton are 7 daily blister strips. Every blister strip contains 4 tablets: 2 for the morning and 2 for the evening (to be taken 12 hours later). When taking ORKAMBI, remove 2 tablets from the blister strip (do not push tablet through the backing).

To help you keep track, you can cut along the blister strip's dotted line to separate your child's morning dose from his or her evening dose. Morning and evening doses contain the same amount of medicine.

## Always take ORKAMBI with food that contains fat

It's important that your child always take ORKAMBI with fat-containing food to help your child's body absorb the medication better.

Your child's healthcare provider can help you choose healthy fat-containing meals and snacks.



Get ideas and recipes for fat-containing meals and snacks in this brochure and at [ORKAMBI.com](http://ORKAMBI.com).

Looking for more new, delicious recipes? Check out [Delicious Dishes](#) for recipe videos.

Evan  
F508del/  
F508del

## If your child misses a dose

**Within 6 hours** of when he or she usually takes it



If your child misses a dose of ORKAMBI<sup>®</sup> (lumacaftor/ivacaftor) and it is **within 6 hours** of when he or she usually takes it, have your child take the dose of ORKAMBI as prescribed with fat-containing food as soon as possible.

**More than 6 hours** after the time when he or she usually takes it



If your child misses a dose of ORKAMBI and it is **more than 6 hours** after the time when he or she usually takes it, have your child skip that dose (only) and take the next dose at the usual time. Do not let your child take 2 doses at the same time to make up for a missed dose.

**More than 1 week** of not taking ORKAMBI



Tell the doctor if your child stops taking ORKAMBI for **more than 1 week**. The doctor may need to change your child's dose of ORKAMBI or other medicines your child may be taking.



## Meet Oscar & Sam

Oscar and his dog Sam explain CF and ORKAMBI in this animated video for you to watch with your child:

[Watch it now.](#)

Please see additional [Important Safety Information](#) on pages 2-4 and full [Prescribing Information](#), including [Patient Information](#).

## Get More From Vertex GPS<sup>™</sup>: Guidance & Patient Support

Vertex GPS provides eligible patients with information about reimbursement and potential financial assistance, product educational resources, and more, including:



**Monthly refill reminders** from your Case Manager



**Delicious Dishes** videos for tasty recipes

Vertex GPS is here to provide you with product support. You can speak to your dedicated Case Manager at **1-877-752-5933 (press 2)**, Monday through Friday, from 8:30 AM to 7:00 PM ET. If your child has been prescribed ORKAMBI<sup>®</sup> (lumacaftor/ivacaftor) and you are not enrolled, please speak with your child's healthcare provider about your interest in Vertex GPS. You can also learn more at [VertexGPS.com](https://VertexGPS.com).

**Please see [Important Safety Information](#) for ORKAMBI on pages 2-4 and full [Prescribing Information](#), including [Patient Information](#).**



ORKAMBI is manufactured for Vertex Pharmaceuticals Incorporated. ORKAMBI, the ORKAMBI logo, Vertex, and the Vertex triangle logo are registered trademarks of Vertex Pharmaceuticals Incorporated. Vertex GPS is a trademark of Vertex Pharmaceuticals Incorporated. All other trademarks referenced herein are the properties of their respective owners.

## RECIPES FOR FAT-CONTAINING MEALS AND SNACKS

Chocolate Chip Pancakes



### INGREDIENTS

- 1¼ cups flour
- 1 tablespoon sugar
- ¼ teaspoon cinnamon
- 1 tablespoon baking powder
- ¼ teaspoon salt
- 2 eggs
- 1 cup whole milk
- 4 tablespoons melted butter
- ¾ teaspoon vanilla
- ½ cup chocolate chips

### DIRECTIONS

1. Preheat griddle or skillet.
2. Combine flour, sugar, cinnamon, baking powder, and salt in a large bowl.
3. Mix together wet ingredients and beat into dry mixture until smooth.
4. Fold in chocolate chips.
5. Pour or spoon batter into griddle or skillet in desired quantity.
6. Flip when top begins to bubble, then cook a minute more.

Source: [GeniusKitchen.com](http://GeniusKitchen.com)

#### 6 SERVINGS

#### NUTRITION PER SERVING

Calories: 268  
Total Fat: 14 g  
Protein: 7 g  
Carbohydrates: 31 g

Chicken Enchilada Casserole



### INGREDIENTS

- 1½ pounds of cooked, chopped chicken
- 1 packet taco powder
- 1 can (15 oz.) enchilada sauce
- 3 cups cooked rice
- 3 cups crushed tortilla chips
- 1 can cream of mushroom chicken soup
- Shredded cheese

### DIRECTIONS

1. Mix the taco powder into the chicken using a little water.
2. Then stir in all other ingredients (except cheese).
3. Pour mixture into a 9x13-inch pan and cover with shredded cheese.
4. Bake at 350°F for 20 minutes. Serve with additional tortilla chips and top with salsa and sour cream.

Source: [CFF.org](http://CFF.org)

#### 12 SERVINGS

#### NUTRITION PER SERVING

Calories: 520  
Total Fat: 10 g  
Protein: 30 g  
Carbohydrates: 85 g

Peanut Butter Balls



### INGREDIENTS

- 1 cup creamy peanut butter
- 1 cup honey
- 2–2½ cups non-fat powdered milk, sifted to remove any lumps

### DIRECTIONS

1. Stir peanut butter and honey together in a large bowl by hand. Gradually add powdered milk.
2. Roll into balls slightly smaller than a walnut. If the mixture is too sticky to roll, add a little more powdered milk.
3. Place on a plate in a single layer and refrigerate for at least 2 hours. Cover with plastic wrap or transfer to an airtight container if you're going to store these for more than a day.

Source: [GeniusKitchen.com](http://GeniusKitchen.com)

#### 8-10 SERVINGS

#### NUTRITION PER SERVING

Calories: 427  
Total Fat: 16 g  
Protein: 19 g  
Carbohydrates: 57 g

## RECIPES FOR FAT-CONTAINING MEALS AND SNACKS

### Blackberry Cobbler



#### INGREDIENTS

- ½ cup butter
- 2 cups self-rising flour
- 2 cups white sugar
- 2 cups milk
- 3½ cups blackberries

#### DIRECTIONS

1. Preheat oven to 350°F. Once oven temperature is reached, melt butter in a 9x13-inch baking pan.
2. In a medium bowl, stir together the flour, sugar, and milk; batter will be slightly lumpy. Pour mixture on top of melted butter in baking pan. Do not mix butter and mixture together.
3. Drop blackberries into batter; if more crust is desired, add less blackberries. Bake in preheated oven for 1 hour or until golden brown.

Source: [AllRecipes.com](http://AllRecipes.com)

11 SERVINGS

#### NUTRITION PER SERVING

Calories: 337  
Total Fat: 10 g  
Protein: 4 g  
Carbohydrates: 60 g

### Ham and Cheese Crescent Roll-ups



#### INGREDIENTS

- 1 (8 oz.) can of pre-made crescent dinner rolls
- 8 thin slices cooked ham
- 4 thin slices cheddar cheese, cut into strips

#### DIRECTIONS

1. Heat oven to 350°F. Separate dough into 8 triangles. Place 1 piece of ham on each triangle; place 2 strips of cheese down center of ham. Fold in edges of ham to match shape of dough triangle.
2. Roll up each crescent, ending at tip of triangle. Place with tips down on ungreased cookie sheet.
3. Bake 15 to 19 minutes or until golden brown. Immediately remove from cookie sheet. Serve warm.

Source: [AllRecipes.com](http://AllRecipes.com)

8 SERVINGS

#### NUTRITION PER SERVING

Calories: 164  
Total Fat: 10 g  
Protein: 6 g  
Carbohydrates: 11 g

### Chocolate Pudding



#### INGREDIENTS

- ½ cup white sugar
- 3 tablespoons unsweetened cocoa powder
- ¼ cup cornstarch
- ⅛ teaspoon salt
- 2¾ cups milk
- 2 tablespoons butter, room temperature
- 1 teaspoon vanilla extract

#### DIRECTIONS

1. In a saucepan, stir together sugar, cocoa, cornstarch, and salt. Place over medium heat, and stir in milk.
2. Bring to a boil and cook, stirring constantly, until mixture thickens enough to coat the back of a metal spoon.
3. Remove from heat, and stir in butter and vanilla.
4. Let cool briefly, and serve warm, or chill in refrigerator until serving.

Source: [AllRecipes.com](http://AllRecipes.com)

4 SERVINGS

#### NUTRITION PER SERVING

Calories: 274  
Total Fat: 10 g  
Protein: 6 g  
Carbohydrates: 43 g