



75/94 mg • 100/125 mg • 150/188 mg oral granules

Every dose matters with ORKAMBI®

A guide for helping your child take ORKAMBI oral granules



Josh
Henry's Dad

Henry
Age 2 years, F508del/F508del

People with cystic fibrosis (CF) pictured may or may not be taking ORKAMBI.

What is ORKAMBI® (lumacaftor/ivacaftor)?

ORKAMBI is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients aged 1 year 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 1 year of age.

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

IMPORTANT SAFETY INFORMATION

Before taking ORKAMBI[®], tell your doctor about all of your medical conditions, including if you:

- have or have had liver problems
- are allergic to ORKAMBI or any ingredients in ORKAMBI. See the Patient Information for a list of ingredients
- have kidney problems
- have lung problems
- have had an organ transplant
- are 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 your doctor about the best birth control method you should use while taking ORKAMBI
- are pregnant or plan to become pregnant. It is not known if ORKAMBI will harm your unborn baby. You and your doctor should decide if you will take ORKAMBI while you are pregnant
- are breastfeeding or planning to breastfeed. It is not known if ORKAMBI passes into your breast milk. You and your doctor should decide if you will take ORKAMBI while you are breastfeeding

Tell your doctor about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

ORKAMBI may affect the way other medicines work, and other medicines may affect how ORKAMBI works. The dose of ORKAMBI may need to be adjusted when taken with certain medicines. Ask your doctor or pharmacist for a list of these medicines if you are not sure.

Especially tell your doctor if you take:

- antibiotics: rifampin (RIFAMATE[®], RIFATER[®]) or rifabutin (MYCOBUTIN[®])
- seizure medicines: phenobarbital, carbamazepine (TEGRETOL[®], CARBATROL[®], and EQUETRO[®]), or phenytoin (DILANTIN[®], PHENYTEK[®])
- sedatives and anti-anxiety medicines: triazolam (HALCION[®]) or midazolam (DORMICUM[®], HYPNOVEL[®], and VERSED[®])
- immunosuppressant medicines: cyclosporine, everolimus (ZORTRESS[®]), sirolimus (RAPAMUNE[®]), or tacrolimus (ASTAGRAF XL[®], ENVARSUS XR[®], PROGRAF[®], and PROTOPIC[®])
- St. John's wort
- antifungal medicines including ketoconazole, itraconazole (such as SPORANOX[®]), posaconazole (such as NOXAFIL[®]), or voriconazole (such as VFEND[®])
- antibiotics including telithromycin, clarithromycin (such as BIAXIN[®]), or erythromycin (such as ERY-TAB[®])

IMPORTANT SAFETY INFORMATION (Continued)

What should I avoid while taking ORKAMBI[®]?

- Do not eat or drink grapefruit products during your first week of treatment with ORKAMBI. Eating or drinking grapefruit products can increase the amount of ORKAMBI in your blood

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 doctor if you have been told you have liver disease as your 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 doctor will do blood tests to check your liver:
 - before you start ORKAMBI
 - every 3 months during your first year of taking ORKAMBI
 - every year while you are taking ORKAMBI

Call your doctor right away if you have any of the following symptoms of liver problems:

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

- **Serious allergic reactions** have happened to people who are treated with ORKAMBI. Call your doctor or go to the emergency room right away if you have any symptoms of an allergic reaction. Symptoms of an allergic reaction may include:
 - rash or hives
 - tightness of the chest or throat or difficulty breathing
 - swelling of the face, lips, and/or tongue, or difficulty swallowing
 - light-headedness or dizziness
- **Breathing problems** such as trouble breathing, shortness of breath or chest tightness in patients when starting ORKAMBI, especially in patients who have poor lung function. Call your doctor right away if you experience these symptoms
- **An increase in blood pressure** in some people receiving ORKAMBI. Call your doctor right away if you have an increase in blood pressure
- **Abnormality of the eye lens (cataract)** in some children and adolescents receiving ORKAMBI. If you are a child or adolescent, your doctor should perform eye examinations before and during treatment with ORKAMBI to look for cataracts

IMPORTANT SAFETY INFORMATION (Continued)

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

Tell your doctor if you have any side effect that bothers you or that does not go away.

These are not all the possible side effects of ORKAMBI. Call your doctor for medical advice about side effects.

You may report side effects to FDA at 1-800-FDA-1088.

Recommended dose of ORKAMBI[®] oral granules

ORKAMBI comes in single-use, oral granule packets for children age 1 through 5 years with cystic fibrosis (CF) who have 2 copies of the F508del-CFTR mutation. The oral granules are similar in size to flour particles and should be mixed with soft food or liquid.

ORKAMBI oral granules are prescribed based on weight and come in 3 different strengths.

AGE	WEIGHT-BASED DOSE	RECOMMENDED DOSE
1 through 2 years old	15 lbs to less than 20 lbs (7 kg to less than 9 kg) lumacaftor 75 mg/ivacaftor 94 mg	1 packet every 12 hours (2 packets per day)
	20 lbs to less than 31 lbs (9 kg to less than 14 kg) lumacaftor 100 mg/ivacaftor 125 mg	
	31 lbs or more (14 kg or more) lumacaftor 150 mg/ivacaftor 188 mg	
2 through 5 years old	Less than 31 lbs (less than 14 kg) lumacaftor 100 mg/ivacaftor 125 mg	
	31 lbs or more (14 kg or more) lumacaftor 150 mg/ivacaftor 188 mg	

Everyday **CF**

Get delicious recipes and food ideas on [Everyday-CF.com](https://www.everyday-cf.com).

How to give your child ORKAMBI® oral granules

STEP 1: Preparation



- Hold the packet with the cut line on top
- Shake the packet gently to settle the ORKAMBI granules
- Tear or cut the packet open along the cut line
- Carefully pour all of the ORKAMBI granules in the packet into **1 teaspoon (5 mL)** of soft food such as flavored pudding or yogurt, or liquid such as juice, in a small container. Food or liquid should be at or below room temperature
- Here are examples of foods and liquids you can mix ORKAMBI granules into. Remember, every child is different, so be sure to talk to your healthcare provider about what the best options are for your child
 - Breast milk
 - Infant formula
 - Puréed fruits or vegetables
 - Yogurt or pudding (flavored, if desired)
 - Applesauce
 - Water
 - Milk
 - Juice
- Then, mix the granules with the 1 teaspoon of food or liquid

STEP 2: Administration



- Within 1 hour of mixing, give ORKAMBI to your child
- Make sure the entire medicine mixture is taken

STEP 3: Fat-containing food before or after the dose



Always take ORKAMBI with fat-containing food

- Make sure to always give your child food that contains fat just before or just after the dose of ORKAMBI oral granules. This helps the body absorb ORKAMBI better

Here are suggestions for fat-containing foods to give your child:

- Breast milk
 - Infant formula
 - Whole milk
 - Whole-milk yogurt
 - Eggs
 - Whole-milk cheese
 - Butter
 - Peanut butter
 - Cheese pizza
 - Avocados
 - Nuts
- Talk to your healthcare provider about fat-containing foods appropriate for your child when taking ORKAMBI granules



Avoid foods and drinks that contain grapefruit during your child's first week taking ORKAMBI because they can increase the amount of ORKAMBI in their body.



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

What if your child misses a dose of ORKAMBI[®]?

- If your child misses a dose of ORKAMBI and it is **within 6 hours** of when they usually take it, have your child take the dose of ORKAMBI as prescribed with fat-containing food as soon as possible
- If your child misses a dose and it is **more than 6 hours** after the time they usually take it, have your child **skip that dose only** and take the next dose with fat-containing food when they usually take it. Do **not** take 2 doses at the same time to make up for a missed dose
- Tell your child's healthcare provider if your child stops taking ORKAMBI for **more than 1 week**. They may need to change your child's ORKAMBI dose or other medicines your child takes



Taking ORKAMBI oral granules with Cammy & Sam

Watch this fun and instructional video with your child for more information on taking ORKAMBI granules at [ORKAMBI.com](https://www.orkambi.com).



Henry,
Age 2 years,
F508del/
F508del

Leisa,
Henry's mom

Tips for helping your child take ORKAMBI[®]

Your child may find that ORKAMBI oral granules have a bitter taste. Every dose matters, so it's important to make sure that your child completely finishes the ORKAMBI mixture at each dose. Here are a few tips that may help.

- 1 Mix the granules with 1 teaspoon (5 mL) of soft food or liquid** that is sweet or rich, like pudding, chocolate sauce, caramel sauce, or melted ice cream.
- 2 Give your child something sweet, like maple syrup or honey**, after they completely finish the dose. It may help with the aftertaste.
- 3 Numb the taste buds before taking ORKAMBI** by having your child eat an ice pop or another cold treat before their dose.
- 4 Put some control in your child's hands** by letting them choose a favorite fat-containing food to eat before or after taking ORKAMBI.
- 5 Does your child have a favorite doll or toy?** Holding a special toy while taking ORKAMBI may make your child feel more comfortable.
- 6 Try creating a rewards system for finishing ORKAMBI, such as collecting stickers.** You know best how to keep your child motivated!



If I mix the ORKAMBI[®] oral granules with a fat-containing food, do I still need to give my child fat-containing food afterwards?

Yes, your child should still eat fat-containing food just before or just after taking the entire mixture.

Does the temperature of the food that I mix with ORKAMBI oral granules matter? Can the granules be mixed in foods that are hot or cold?

The granules should be mixed with soft food or liquid at room temperature or below. The granules should not be mixed in items that are frozen or hot.

Can my child swallow the granules without mixing them in soft food or liquid?

The entire contents of each packet should be mixed with 1 teaspoon (5 mL) of age-appropriate soft food or liquid. The mixture should be taken within 1 hour of being mixed. Make sure all medicine is taken.

My child vomited after taking the dose of ORKAMBI oral granules. What should I do?

You should contact your child's healthcare provider to discuss this. Your pharmacist may also be a good resource.

If my child is napping when it is time for ORKAMBI oral granules, should I wait until they're awake to administer them?

You should give your child ORKAMBI oral granules every 12 hours or as prescribed by your child's healthcare provider.

Do the granules have a taste?

Your child may find that ORKAMBI[®] granules have a bitter taste. To ensure your child takes the entire dose, try mixing it into a food that is sweet or rich, like pudding, chocolate sauce, caramel sauce, or melted ice cream. You can also try giving your child something sweet, like maple syrup or honey, after the dose. Another tip is to numb the taste buds by giving your child an ice pop or cold treat before the dose of ORKAMBI. [For more tips, see page 8.](#)

How should ORKAMBI oral granules be stored?

ORKAMBI should be stored at 68°F to 77°F (20°C to 25°C). Do not use ORKAMBI after the expiration date on the package. Keep ORKAMBI and all medicines out of the reach of children.

How are ORKAMBI oral granules packaged?

Every carton has 56 ORKAMBI oral granule packets, organized in 4 weekly wallets. Each wallet has 2 packets for each of the 7 days in a week.

Are the oral granules packets childproof?

The packets are child-resistant.

What should my child avoid while taking ORKAMBI?

Avoid foods and drinks that contain grapefruit during your child's first week taking ORKAMBI because they can increase the amount of ORKAMBI in their body.

Meet Cammy and her dog, Sam



Cammy and Sam are here to explain how to prepare and take ORKAMBI[®] oral granules.

Watch Taking ORKAMBI oral granules with Cammy & Sam



For more information about how to take ORKAMBI, visit [ORKAMBI.com](https://www.orkambi.com) and watch this fun and informative video.

Color Cammy and Sam's world



[Download coloring pages](#) as a fun activity for your child. You can hang their latest masterpiece on the wall or refrigerator.



We're here to help you get there

Wherever life with cystic fibrosis (CF) takes you, Vertex GPS™: Guidance & Patient Support is here to help. We offer personalized, one-on-one support to help you start and stay on track with treatment. Once you're enrolled, you'll be assigned a dedicated Support Specialist who will be with you every step of the way.

Here are just some of the ways your Support Specialist can help:



Get you started on treatment by verifying your coverage and out-of-pocket costs with your **insurance company**. They'll also connect with your **healthcare provider** to discuss any requirements or questions your insurance company may have while determining coverage.



Help you explore financial assistance options, regardless of your insurance coverage. And if you have commercial insurance, the Vertex GPS Co-pay Assistance Program may be able to lower your co-pay to as little as \$0 per fill.*

*Eligibility restrictions and limitations apply. Annual assistance is limited to a maximum of \$20,000.



Keep you on track with your treatment by coordinating shipments with your **specialty pharmacy** and reminding you when it's time to refill your Vertex medicine. And if your daily routine changes, they can help you pre-plan refills, ship your medicine to a new address, and share tips to help you stay motivated.



Meet your everyday needs with information on nutrition and tips for staying physically active and maintaining a healthy mindset. And if you're caring for someone with CF, they'll send educational resources to help you teach your loved one about the importance of their daily treatment routine.



Plan for what's ahead as you approach big life changes. They can help you prepare for your next chapter and give you tips on staying on track with treatment. They can also share advice from others living with CF.



Not enrolled in Vertex GPS?

If you have been prescribed a Vertex medicine, ask your healthcare provider to complete an enrollment form for you.



Already enrolled?

If you are currently enrolled in GPS, you can call or text your Support Specialist at **1-877-752-5933 (press 2 when calling)**, Monday through Friday, from 8:30 AM to 7 PM ET.



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Discover more about GPS and the support resources available at VertexGPS.com.