ORKAMBI®

Consumer Medicine Information (CMI) summary

The full CMI on the next page has more details. If you are worried about using this medicine, speak to your doctor or pharmacist.

1. Why am I using ORKAMBI?

ORKAMBI contains the active ingredients lumacaftor and ivacaftor. ORKAMBI is used for the treatment of cystic fibrosis (CF) in patients aged 1 year or older who are homozygous for the *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene. For more information, see Section 1. Why am I using ORKAMBI? in the full CMI.

2. What should I know before I use ORKAMBI?

Do not use if you have ever had an allergic reaction to ORKAMBI or any of the ingredients listed at the end of the CMI.
Talk to your doctor if you have any other medical conditions, take any other medicines, or are pregnant or plan to become pregnant or are breastfeeding.
For more information, see Section 2. What should I know before I use ORKAMBI? in the full CMI.

3. What if I am taking other medicines?

Some medicines may interfere with ORKAMBI and affect how it works.
A list of these medicines is in Section 3. What if I am taking other medicines? in the full CMI.

4. How do I use ORKAMBI?

- Your doctor will tell you how much ORKAMBI you need to take each day. This may depend on your condition and whether or not you are taking any other medicines. ORKAMBI is for oral use.

More instructions can be found in Section 4. How do I use ORKAMBI? in the full CMI.

5. What should I know while using ORKAMBI?

Things you should do	• Remind any doctor, dentist or pharmacist you visit that you are using ORKAMBI. • Use ORKAMBI exactly as your doctor has prescribed. • Take ORKAMBI with fat containing food. • Tell your doctor if you become pregnant or plan to become pregnant while using ORKAMBI.
Things you should not do	• Do not stop using ORKAMBI or change the dose without first checking with your doctor. • Do not take any other medicines whether they require a prescription or not without first telling your doctor or consulting a pharmacist.
Looking after your medicine	• Store below 30°C, in a dry place away from moisture, heat or sunlight. • Keep this medicine where young children cannot reach it.

For more information, see Section 5. What should I know while using ORKAMBI? in the full CMI.

6. Are there any side effects?

All medicines can have side effects. Sometimes they are serious, most of the time they are not. Tell your doctor straight away if you feel unwell while using ORKAMBI. Some side effects may need medical attention.

Common side effects include shortness of breath, diarrhoea and nausea. You may experience changes in mood or behaviour such as anxiety, depression, suicidal thoughts and behaviours, and trouble sleeping. If you experience significant pain or discomfort in the upper right stomach (abdominal) area, yellowing of your skin or the white parts of your eyes, loss of appetite, nausea, vomiting, dark urine or confusion this may indicate problems with your liver. If you experience rash, tightness of the chest or throat, or difficulty breathing, swelling of the face, lips and/or tongue, difficulty swallowing or dizziness, these may be serious side effects associated with an allergic reaction. If you experience any of these reactions, you should see your doctor straight away or go to the emergency department at your nearest hospital.

For more information, including what to do if you have any side effects, see Section 6. Are there any side effects? in the full CMI

ORKAMBI® (or-KAM-bee)

Active ingredient(s): *Lumacaftor/ Ivacaftor*

Consumer Medicine Information (CMI)

This leaflet provides important information about using ORKAMBI. **You should also speak to your doctor or pharmacist if you would like further information or if you have any concerns or questions about using ORKAMBI.**

Where to find information in this leaflet:

1. Why am I using ORKAMBI?
2. What should I know before I use ORKAMBI?
3. What if I am taking other medicines?
4. How do I use ORKAMBI?
5. What should I know while using ORKAMBI?
6. Are there any side effects?
7. Product details

1. Why am I using ORKAMBI?

ORKAMBI contains the active ingredients lumacaftor and ivacaftor.

ORKAMBI is used for the treatment of cystic fibrosis (CF) in patients aged 1 years or older who are homozygous for the *F508del*mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene.

What is cystic fibrosis?

Cystic fibrosis is caused by genetic defects that limit the flow of chloride and water through cell membranes. As a result, the mucus in the lungs (and other organs) becomes thick and sticky, clogs the lungs and makes it easier for germs to grow. ORKAMBI is a medicine that works by improving the flow of chloride and water in patients with cystic fibrosis who have a certain genetic defect.

ORKAMBI helps your breathing by improving your lung function. You may also notice that you do not get ill as often, or that it is easier to maintain a healthy weight.

How ORKAMBI works

ORKAMBI belongs to a group of medicines called "cystic fibrosis transmembrane conductance regulator (*CFTR*) modulators".

The *CFTR* protein is a chloride ion channel that is normally present at the cell surface of epithelial cells where it transports chloride ions out of the cell in multiple organs. In people who have two copies of the *F508del* mutation in the *CFTR* gene, the amount of *CFTR* protein at the cell surface is much lower than normal. The small amount of *CFTR* protein that reaches the cell surface does not function properly, as the chloride ion channel remains closed more often than normal.

ORKAMBI is a combination product that contains a *CFTR* corrector (lumacaftor) which increases the amount of working *CFTR* at the cell surface, and a *CFTR* potentiator (ivacaftor), which makes the *CFTR* protein at the cell surface function better because the chloride channel is open more often.

ORKAMBI therefore allows more chloride ions to get transported out of cells in multiple organs.

ORKAMBI is not addictive.

2. What should I know before I use ORKAMBI?

Warnings

Do not use ORKAMBI if:

- you are allergic to any medicine containing lumacaftor or ivacaftor, the active ingredients in ORKAMBI, or any of the ingredients listed at the end of this leaflet.
- Always check the ingredients to make sure you can use this medicine.

Check with your doctor if you:

- have any other medical conditions
- take any medicines for any other condition (see Section 3. What if I am taking other medicines?)

During treatment, you may be at risk of developing certain side effects. It is important you understand these risks and how to monitor for them. See additional information under Section 6. Are there any side effects?

Pregnancy and breastfeeding

Check with your doctor if you are pregnant or intend to become pregnant.

Talk to your doctor if you are breastfeeding or intend to breastfeed. Lumacaftor and ivacaftor have been detected in breast milk. If you plan to breastfeed, ask your doctor for advice before taking ORKAMBI.

Check with your doctor if you are using or plan to use hormonal birth control (oral, injectable, skin patches and implantable products) as this type of birth control may not work when taking ORKAMBI (see Section 3. What if I am taking other medicines?)

Use in children

Do not give ORKAMBI to children under 1 year of age.

It is not known if ORKAMBI is safe and effective in children under 1 year of age.

Tell your doctor if:

- you have been told you have liver or kidney disease.
- you experience low mood or any other changes in mood and behaviour (such as anxiety, low or altered mood, suicidal thoughts or behaviours, or problems with sleep).
- your child displays behaviours that are different to their usual patterns whilst taking ORKAMBI.

Worsening of liver function has been seen in patients with severe liver disease and can be serious or fatal.

Laboratory testing

Your doctor will do some blood tests to check your liver before you start taking and while you are taking ORKAMBI, particularly in the first year (every 3 months) and once a year after that.

Abnormal blood tests of the liver have been seen in some people receiving ORKAMBI. If you have had abnormal blood tests of the liver in the past, your doctor may adjust your dose of ORKAMBI and may order blood tests to test your liver more often.

Other testing

Lungs

Respiratory events such as shortness of breath or chest tightness were seen in patients when starting ORKAMBI, especially in patients who have poor lung function. If you have poor lung function, your doctor may monitor you more closely when you start ORKAMBI.

Blood Pressure

An increase in blood pressure has been seen in some patients treated with ORKAMBI. Your doctor may monitor your blood pressure during treatment with ORKAMBI.

Eyes

Abnormality of the eye lens (cataract) without any effect on vision has been noted in some children receiving ORKAMBI. Your doctor may perform eye examinations before and during treatment with ORKAMBI.

3. What if I am taking other medicines?

Tell your doctor or pharmacist if you are taking any other medicines, including any medicines, vitamins or supplements that you buy without a prescription from your pharmacy, supermarket or health food shop.

Some medicines may be affected by ORKAMBI or may affect how well it works. You may need different amounts of your medicines, or you may need to take different medicines. Your doctor will advise you.

Tell your doctor or pharmacist if you are taking any of the following:

Anti-allergic medicines *used for the relief and treatment of allergies*	montelukast, fexofenadine
Antifungal medicines *used for the treatment of fungal infections*	ketoconazole, itraconazole, posaconazole, voriconazole, fluconazole
Anti-allergic medicines *used for the relief and treatment of allergies*	montelukast, fexofenadine
Antibiotic medicines *used for treatment of bacterial infections*	telithromycin, clarithromycin, erythromycin, rifampicin, rifabutin, rifapentine
Anticonvulsant medicines *used for the treatment of epileptic seizures*	phenobarbital, carbamazepine, phenytoin
Anti-inflammatory medicines *used for the treatment of pain associated with inflammation*	ibuprofen
Herbal medicine	St. John's wort *(Hypericum perforatum)*
Benzodiazepines *used for the treatment of anxiety, insomnia, agitation etc*	midazolam, triazolam
Immunosuppressants *used after organ transplantation*	ciclosporin, everolimus, sirolimus, tacrolimus
Cardiac glycosides *used for the treatment of mild to moderate congestive heart failure and an abnormal heart rhythm*	digoxin
Anticoagulants *used to prevent blood clots from forming or growing larger in blood and blood vessels*	warfarin, dabigatran
Hormonal contraceptives *used to prevent pregnancy*	oral, injectable, skin patches (transdermal) and implantable products
Glucocorticoids *used to treat inflammation*	prednisone, methylprednisolone
Antidepressants *used to treat depression*	citalopram, escitalopram, sertraline, bupropion
Oral hypoglycemics *used to treat Type 2 diabetes*	repaglinide
Medicines *used to treat acid reflux disease and ulcers*	Proton pump inhibitors such as omeprazole, esomeprazole, lansoprazole, H2 blockers such as ranitidine

Your doctor or pharmacist has more information on medicines to be careful with or to avoid while taking ORKAMBI.

Check with your doctor or pharmacist if you are not sure about what medicines, vitamins or supplements you are taking and if these affect ORKAMBI.

4. How do I use ORKAMBI?

How much to take/use

Your doctor will tell you how much ORKAMBI you need to take each day. This may depend on your condition and whether or not you are taking any other medicines.

The recommended dose of ORKAMBI for patients aged 1 year and older is as follows:

Patients aged 1 to less than 2 years and weighing 7 kg to less than 9 kg:

Morning: One sachet of 75 mg lumacaftor/94 mg ivacaftor granules taken orally.

Evening (12 hours later): One sachet of 75 mg lumacaftor/94 mg ivacaftor granules taken orally.

Patients aged 1 to less than 2 years and weighing 9 kg to less than 14 kg:

Morning: One sachet of 100 mg lumacaftor/125mg ivacaftor granules taken orally.

Evening (12 hours later): One sachet of 100 mg lumacaftor/125 mg ivacaftor granules taken orally.

Patients aged 1 to less than 2 years and weighing less than or equal to 14 kg:

Morning: One sachet of 150 mg lumacaftor/188 mg ivacaftor granules taken orally.

Evening (12 hours later): One sachet of 150 mg lumacaftor/188 mg ivacaftor granules taken orally.

Patients aged 2 to 5 years and weighing less than 14 kg:

- One sachet of 100 mg lumacaftor/125 mg ivacaftor granules taken orally every 12 hours (total daily dose of 200mg lumacaftor/ 250mg ivacaftor).

Patients aged 2 to 5 years and weighing greater than or equal to 14 kg:

- One sachet of 150 mg lumacaftor/ 188 mg ivacaftor granules taken orally every 12 hours (total daily dose of 300 mg lumacaftor/ 376 mg ivacaftor).

Patients 6-11 years:

- Two tablets (100 mg lumacaftor/125 mg ivacaftor in each tablet) taken orally every 12 hours (total number of tablets is 4 per day for a total of 400 mg lumacaftor/500 mg ivacaftor).

Patients 12 years and over:

- Two tablets (200 mg lumacaftor/125 mg ivacaftor in each tablet) taken orally every 12 hours (the total number of tablets is 4 per day for a total of 800 mg lumacaftor/500 mg ivacaftor).

When to take / use ORKAMBI

- ORKAMBI should be used every day for as long as your doctor tells you to.
- Your doctor will determine if your treatment should be stopped.

If you have liver problems, your doctor may need to reduce your dose of ORKAMBI.

How to take ORKAMBI

ORKAMBI is for oral use.

Take ORKAMBI with fat-containing food.

Examples of meals or snacks that contain fat are those prepared with butter or oils or those containing eggs.

Other fat-containing foods are:

- Cheese, whole milk, breast milk, infant formula, whole-milk dairy products, yoghurt, chocolate
- Meats, oily fish
- Eggs
- Avocados, soy-based products (tofu)
- Nuts

Taking ORKAMBI with fat-containing food is important to get the right levels of medicine in your body.

Tablets:

Take 2 tablets of ORKAMBI every 12 hours by mouth with a fat-containing meal or snack.

Granules:

Each sachet is for single use only. Take one sachet of ORKAMBI every 12 hours.

Giving ORKAMBI to your child:

- Hold sachet of granules with cut line on top.
- Shake sachet gently to settle contents.
- Tear or cut sachet open along cut line.
- Mix the entire content of a sachet with 5 mL of age-appropriate soft food or liquid. Food or liquid should be at room temperature or below. Some examples of age-appropriate soft foods or liquids include puréed fruits or vegetables, yogurt, water, milk, breast milk, infant formula or juice.
- Once mixed, give the product to your child immediately. If this is not possible, give it within one hour after mixing. Ensure that the mixture is completely and immediately consumed.
- A fat-containing meal or snack should be given to your child just before or just after dosing.

If you forget to use ORKAMBI

ORKAMBI should be used regularly at the same time each day.

If less than 6 hours have passed since you missed a dose, take the dose as soon as possible and then go back to your regular schedule.

If more than 6 hours have passed since you missed a dose then skip the dose you missed and take your next dose when you are meant to.

Do not take a double dose to make up for the dose you missed. This may increase the chance of you getting an unwanted side effect.

If you are not sure what to do, talk to your doctor or pharmacist.

If you use too much ORKAMBI

If you think that you have used too much ORKAMBI, you may need urgent medical attention.

You should immediately:

- phone the Poisons Information Centre

  (by calling 13 11 26), or
- contact your doctor, or
- go to the Emergency Department at your nearest hospital.

You should do this even if there are no signs of discomfort or poisoning.

5. What should I know while using ORKAMBI?

Things you should do

Keep all of your doctor's appointments so that your progress can be checked.

- Use ORKAMBI exactly as your doctor has prescribed.
- Take ORKAMBI with fat-containing food
- Tell your doctor if you become pregnant while using ORKAMBI.
- Tell your doctor if, for any reason, you have not used ORKAMBI exactly as prescribed.
- Tell your doctor or pharmacist as soon as possible if you do not feel well while taking ORKAMBI.

Things you should not do

- Do not stop using ORKAMBI or change the dose without first checking with your doctor.
- Do not let yourself run out of medicine over the weekend or on holidays.
- Do not give ORKAMBI to anyone else even if they have the same condition as you.
- Do not use ORKAMBI to treat other complaints unless your doctor tells you to.
- Do not start taking any other medicines, whether they require a prescription or not without telling your doctor first or consulting a pharmacist.

Driving or using machines

Be careful before you drive or use any machines or tools until you know how ORKAMBI affects you.

Looking after your medicine

- Store below 30ºC
- **Keep it where young children cannot reach it.**

Store it in a cool dry place away from moisture, heat or sunlight; for example, do not store it:

- in the bathroom or near a sink, or
- in the car or on window sills.

Getting rid of any unwanted medicine

If your doctor tells you to stop using ORKAMBI or it is out of date, take it to any pharmacy for safe disposal.

Do not use this medicine after the expiry date.

6. Are there any side effects?

All medicines can have side effects. If you do experience any side effects, most of them are minor and temporary. However, some side effects may need medical attention.

See the information below and, if you need to, ask your doctor or pharmacist if you have any further questions about side effects.

Less serious side effects

Less serious side effects	What to do
Very common side effects (may affect more than 1 in 10 people) • shortness of breath • diarrhoea • nausea **Common side effects (may affect up to 1 in 10 people)** • chest tightness • sore throat • upper respiratory tract infection • gas • rash • stuffy or runny nose • vomiting • irregular periods (menses) or pain with menses **Uncommon side effects (may affect up to 1 in 100 people)**	**Speak to your doctor if you have any of these less serious side effects and they worry you.**

Less serious side effects	What to do
• abnormal or irregular periods, including the absence or infrequent menses, or more frequent or heavier menstrual bleeding • increase in blood pressure **Additional side effects in children:** • cough with sputum • stuffy nose • headache • stomach pain • increase in sputum	

Serious side effects

Serious side effects	What to do
The side effects listed below may indicate problems with your liver. You may need urgent medical attention if you experience: • significant pain or discomfort in the upper right stomach (abdominal) area. • yellowing of your skin or the white parts of your eyes. • loss of appetite • nausea or vomiting • dark urine • confusion. **The side effects listed below may indicate a serious allergic reaction. You may need urgent medical attention if you experience:** • rash or hives • tightness of the chest or throat or difficulty breathing • swelling of the face, lips and/or tongue, difficulty swallowing • light-headedness or dizziness. **The side effects listed below may indicate a mood disturbance. Tell your doctor if you experience:** • depression • suicidal thoughts • anxiety • sleep disturbance • behavioural changes in young children (2-5 yrs).	**Call your doctor straight away, or go straight to the Emergency Department at your nearest hospital if you notice any of these serious side effects.**

Tell your doctor or pharmacist if you notice anything else that may be making you feel unwell.

Other side effects not listed here may occur in some people.

Reporting side effects

After you have received medical advice for any side effects you experience, you can report side effects to the Therapeutic Goods Administration online at www.tga.gov.au/reporting-problems. By reporting side effects, you can help provide more information on the safety of this medicine.

Always make sure you speak to your doctor or pharmacist before you decide to stop taking any of your medicines.

7. Product details

This medicine is only available with a doctor's prescription.

What ORKAMBI contains

Tablets:

Active ingredients (main ingredient)	lumacaftor ivacaftor
Other ingredients (inactive ingredients)	microcrystalline cellulose croscarmellose sodium hypromellose acetate succinate povidone sodium lauryl sulfate magnesium stearate OPADRY II Pink 85F140026 (PI# 110227) OPACODE monogramming ink S-1-17823 BLACK (PI# 12108)

Granules:

Active ingredients (main ingredient)	lumacaftor ivacaftor
Other ingredients (inactive ingredients)	microcrystalline cellulose hypromellose acetate succinate croscarmellose sodium sodium lauryl sulfate povidone

Do not take this medicine if you are allergic to any of these ingredients.

What ORKAMBI looks like

ORKAMBI 100/125 mg Tablets (AUST R 289715):

- Pink, oval-shaped tablets, printed with "1V125" in black ink on one side and plain on the other.

ORKAMBI 200/125 mg Tablets (AUST R 235759):

- Pink, oval-shaped tablets, printed with "2V125" in black ink on one side and plain on the other.

ORKAMBI 75/94 mg Granules (AUST R 389450),

ORKAMBI 100/125 mg Granules (AUST R 305011),

and ORKAMBI 150/188 mg Granules (AUST R 305012)

- White to off-white granules enclosed in unit dose sachets

Availability

Tablets:

ORKAMBI tablets are available in the following pack sizes:

- 112-count tablet box (contains a 4-week supply in 4 weekly cartons of 7 daily blister strips with 4 tablets per strip)

ORKAMBI tablets are available in the following strength:

- 200 mg of lumacaftor and 125 mg of ivacaftor
- 100 mg of lumacaftor and 125 mg of ivacaftor

Granules:

ORKAMBI granules are available in the following pack size:

- 56 sachets (containing 4 individual wallets with 14 sachets per wallet)

ORKAMBI granules are available in the following strengths:

- 75 mg of lumacaftor and 94 mg of ivacaftor
- 100 mg of lumacaftor and 125 mg of ivacaftor
- 150 mg of lumacaftor and 188 mg of ivacaftor

Who distributes ORKAMBI

Vertex Pharmaceuticals (Australia) Pty Ltd

Suite 3, Level 3

601 Pacific Highway

St Leonards NSW 2065

Australia

Telephone: 1800 179 987

e-mail: VertexMedicalInfo@vrtx.com

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