

Orkambi FAQ

1. What is Orkambi and who can take the drug?

Orkambi is the brand name for the combination of ivacaftor (Kalydeco™) and lumacaftor. It is the first Health Canada approved treatment that targets the underlying cause of CF in people with two copies of the F508del mutation, the most common CF mutation.

Orkambi is taken in pill form every 12 hours —once in the morning and once in the evening.

2. What CF mutations does Orkambi treat?

Orkambi is designed for people with two copies of the most common CF mutation, F508del.

Health Canada has approved the drug only for people with two copies of F508del. This applies to approximately 55 % of CF patients in Canada.

3. How does Orkambi work?

Orkambi is designed to help the defective CFTR protein function more normally.

The drug is a combination of ivacaftor (Kalydeco™) and lumacaftor, two separate drugs taken as one pill. Both drugs are designed to address the underlying cause of CF —a protein, called CFTR, that is defective because of the mutations in the CF gene.

In people with two copies of F508del, a series of problems prevents the CFTR protein from reaching the cell surface, where it is needed to help regulate the flow of salt and fluids in and out of the cells of the lungs and other organs. The small amount that gets to the cell surface does not function properly and turns over more rapidly than normal.

Lumacaftor is designed to help move some of the defective CFTR protein to its proper place at the cell surface. Ivacaftor then increases the activity of the protein once it is there, supporting the flow of salt and fluids.

In the study of 1100 people with two copies of the F508del mutation, Orkambi showed a modest but statistically significant improvement in percent predicted FEV₁ (about 4%).

In the same study, compared with the placebo group, pulmonary exacerbations were significantly reduced (about 30-39%). Small improvements in body mass index (BMI) and a quality of life measure were also reported.

Some patients do not respond to Orkambi, or may experience uncomfortable side effects such as shortness of breath.

4. Does Orkambi work in people with one copy of F508del?

Orkambi was tested in people with CF with only one copy of the F508del mutation and was shown not to be effective.

5. Will people with CF who take Orkambi still need other therapies?

YES. Yes yes yes!

While Orkambi may be an important new treatment for some people with two copies of F508del, patients on Orkambi will still need all their daily treatments (medications, exercise, airway clearance, enzymes) to help manage the symptoms of the disease. Talk with your CF care team about how Orkambi may or may not fit into your treatment regimen.

6. Is Orkambi a cure for cystic fibrosis?

No. While Orkambi improves lung function and other important health measures in people with two copies of the F508del mutation, it is not a cure. People with CF who take the combination drug will still need other daily treatments to manage the symptoms of the disease. Further studies are needed to determine the longer term impact of this medication on the natural history of CF.

7. How can I find out which CF mutations I have?

If you do not know your CF mutations, contact your CF Clinic. If the information is not already in your patient file, testing can be arranged through the clinic.

8. How much does this medication cost, when will it be available, and what coverage is available?

Orkambi is expected to cost around \$300,000CAN per year. We do not know the exact figure at this time.

As this medication has only just been approved (Jan. 26, 2016) by Health Canada, there is not presently a supply available locally. This is expected to take about a month.

BC Pharmacare does not cover this drug. Negotiations around government funding for this medication have not yet started and are likely to be lengthy. Approval for Kalydeco took about 2 years, so it would be reasonable to expect a similar timeline in this case. It is also worth noting that the negotiation (when and if it takes place) may not result in an agreement to cover the drug. Other jurisdictions (notably New Zealand) have declined to cover this medication.

Some third party insurance policies MAY cover Orkambi. This will vary between individual companies and plans. Please check with your third party insurance provider if you are unsure if you would be covered.

Any information required by your provider can be provided by your CF Clinic with your approval. The pharmaceutical company will also be sponsoring a service that may help to streamline this process.

As always, if you have any questions about this or any other aspect of your CF care, please contact the CF Clinic. We are always happy to discuss your health care with you.

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