ARIZONA PEDIATRIC PULMONARY CENTER AUGUST 2015

NEWSLETTER

A CF MEDICATION UPDATE: ORKAMBI® (LUMACAFTOR/IVACAFTOR)

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What is Orkambi® (lumacaftor/ivacaftor) and how does it work?

Orkambi® (lumacaftor/ivacaftor) is an oral medication that was recently FDA approved for use in patients 12 years and older with cystic fibrosis who carry a specific mutation. The two medications in Orkambi® (lumacaftor/ivacaftor) offer a dual mechanism that both stabilizes the chloride channels by bringing more to the cell surface and allowing them to be open for a longer period of time. This allows for better flow of salt and water across membranes, leading to better hydration and decreased mucus in the lungs.

Which CF mutation(s) is Orkambi® (lumacaftor/ivacaftor) approved for? How do I find out about which CF mutations I have?

Patients with two copies of the F508del mutation are eligible for Orkambi® (lumacaftor/ivacaftor). If you had genetic testing done previously, the CF Team should have your CF-specific mutations available in your medical records. If you have not had genetic testing done, ask the CF Team to discuss arrangements for testing.

How should this medication be taken?

Each tablet contains 200 mg of lumacaftor and 125 mg of ivacaftor. The normal dose, for patients ages 12 years and older, is 2 tablets taken by mouth twice daily. Each dose should be taken with a high-fat meal; around 20 g of fat is recommended.

If I am started on Orkambi® (lumacaftor/ivacaftor), what can I expect as far as possible side effects and necessary monitoring by the CF Team?

Orkambi® (lumacaftor/ivacaftor) is broken down by the liver and may lead to an increase in liver enzymes. Before you begin this medication, initial tests to check your liver enzymes (a blood test) will be performed. During the first year of therapy, these will be checked every three months. If they continue to remain in the normal range at one year of treatment, then once a year checks are enough.

For patients under 18 years of age, there may be a higher risk of developing cataracts while on Orkambi® (lumacaftor/ivacaftor). It is recommended that these patients receive an eye exam before starting therapy and then annually, to catch these early if they do occur.

Finally, around 10% of patients in the trial experienced a sensation of chest-tightness or shortness-of-breath while on Orkambi® (lumacaftor/ivacaftor). However, this sensation was not associated with any significant harm or loss of lung function. For most patients who continued taking the medicine, this sensation resolved over time. If you experience this while taking Orkambi® (lumacaftor/ivacaftor), please notify the CF Team as soon as possible.

This medication can have significant interactions with other medications. It is important to remember to always let your CF pharmacist know if you plan on starting any new medications (over-the-counter or prescription) as they may interact with Orkambi® (lumacaftor/ivacaftor).

How soon can I start taking Orkambi® (lumacaftor/ivacaftor) if I qualify?

Because of the cost of this medication, additional paperwork may be required for insurance providers to approve its use. After your initial labs/screening and visit with the CF Team to discuss Orkambi® (lumacaftor/ivacaftor), we will begin the process for securing insurance approval. This may take days to weeks depending on what documentation each type of insurance may need. Please note that they will require proof of CF mutation type, so if original lab results are not available in your medical record, this lab test may need to be performed again. After insurance approves the use of Orkambi® (lumacaftor/ivacaftor), the CF Team will prescribe enough medicine for 4 months. This will provide enough of a supply until you can recheck your liver enzymes after 3 months of therapy. After checking your lab results and attending your quarterly follow-up appointment, you will receive a new prescription. This process of an updated prescription, every 3 to 4 months after labs and follow up, will occur for the first year of therapy.

What if I have questions about Orkambi® (lumacaftor/ivacaftor)?

For any questions pertaining to your medications, including Orkambi® (lumacaftor/ivacaftor), please feel free to contact the CF Center's Pharmacist, Hanna Phan, PharmD. She can be reached via contacting the CF Center nurse, Veronica Cruz, RN by phone (520-626-9988) or through MyChart.

ONLINE RESOURCES

www.uappc.peds.arizona.edu
The Pediatric Pulmonary Center's website

www.CysticLife.org
A social network for the cystic fibrosis community

UPCOMING EVENTS

The Monster Mash & 5k Dash
4:30 PM, October 18, 2015
Gentle Ben's

Breath of Life Gala
6:00 PM, November 21, 2015
Loew's Ventana Resort

CONTACT INFORMATION

Medical Questions
(520)694-5132

Newsletter Questions
(520)626-2962

ATTENTION PATIENTS, PARENTS, AND CAREGIVERS

We are in the beginning process of creating a **CF Patient/Family Advisory Board**. We are looking for motivated and committed individuals who are able to volunteer their time to participate in a monthly CF Family Advisory Board meeting. We want to invite your leadership and feedback into CF care provided, and encourage your participation in planning events specific to CF patient and family care, and CF based research projects. If this is something you would be interested in getting involved with, please contact me by **September 30, 2015** with your contact information and any time/date constraints.

Thank you,
Mary McGuire | mmcguire@peds.arizona.edu | 520-626-1569

QUALITY IMPROVEMENT INITIATIVE

Our CF care team was selected as one of fifteen CF centers across the country to participate in a quality improvement (QI) initiative through the CF Foundation. This particular quality improvement program is entitled, "Fundamentals Learning and Leadership Collaborative". Our CF team has weekly QI meetings with our CF Foundation designated QI coach, and included in our QI team is a CF patient and a CF parent. We are focused on working together and learning with our patients and families to produce the best possible care for those living with CF. We will provide regular updates on our QI initiatives through this CF quarterly newsletter, and updates will also be posted in the hall outside the clinic rooms on CF clinic days. We are currently in the process of evaluating the care provided and choosing an area of focus based upon the data received. Thank you to those who have completed surveys in clinic. We are excited for this opportunity to work together to improve CF care and welcome your feedback.

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