CareSource Rxinnovations

# PHARMACY POLICY STATEMENT
## Marketplace

| DRUG NAME | Orkambi (lumacaftor/ivacaftor) |
|---|---|
| BILLING CODE | Must use valid NDC |
| BENEFIT TYPE | Pharmacy |
| SITE OF SERVICE ALLOWED | Home |
| STATUS | Prior Authorization Required |

Orkambi, initially approved by the FDA in 2015, is a combination of lumacaftor and ivacaftor, a cystic fibrosis transmembrane conductance regulator (CFTR) potentiator, indicated for the treatment of cystic fibrosis in patients age 2 years and older who are homozygous for the F508del mutation in the CFTR gene. Cystic fibrosis is an autosomal recessive disease in which patients can have abnormal airways secretions, chronic endobronchial infection, and progressive airway obstruction.

Orkambi (lumacaftor/ivacaftor) will be considered for coverage when the following criteria are met:

## Cystic Fibrosis

For **initial** authorization:

1. Member is at least 2 years of age; AND
2. Medication must be prescribed by or in consultation with a pulmonologist or an infectious disease specialist; AND
3. Member has a diagnosis of cystic fibrosis; AND
4. Member has had genetic testing documented in chart notes with two copies (homozygous) of the F508del mutation (F508del/F508del) in their CFTR gene; AND
5. **Dosage allowed/Quantity limit:** (112 tablets per 28 days or 56 unit-dose packets per 28 days)
   a) Adults and pediatric members age 12 years and older: two tablets (each containing lumacaftor 200 mg/ivacaftor 125 mg) taken orally every 12 hours.
   b) Pediatric members age 6 through 11 years: two tablets (each containing lumacaftor 100 mg/ivacaftor 125 mg) taken orally every 12 hours.
   c) Pediatric members age 2 through 5 years < 14 kg: one packet of granules (each containing lumacaftor 100 mg/ivacaftor 125 mg) every 12 hours
   d) Pediatric members age 2 through 5 years ≥ 14 kg or greater: one packet of granules (each containing lumacaftor 150 mg/ivacaftor 188 mg) every 12 hours

***If all the above requirements are met, the medication will be approved for 3 months.***

For **reauthorization**:

1. Member must be in compliance with all other initial criteria; AND
2. Member's adherence to medication is confirmed by claims history; AND
3. Chart notes must show improvement or stabilized signs and symptoms of disease demonstrated by any of the following:
   a) Improved FEV1 and/or other lung function tests
   b) Improvement in sweat chloride
   c) Decrease in pulmonary exacerbations
   d) Decrease in pulmonary infections
   e) Increase in weight-gain

f) Decrease in hospitalizations

***If all the above requirements are met, the medication will be approved for an additional 12 months***

## CareSource considers Orkambi (lumacaftor/ivacaftor) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

| DATE | ACTION/DESCRIPTION |
|---|---|
| **06/12/2017** | New policy for Orkambi created. Not covered diagnosis added. |
| **03/14/2019** | Age coverage expanded (approved for 2 years old members and older). |
| **12/30/2020** | Diagnosis of cystic fibrosis added to initial criteria. Reauthorization criteria updated to ask for evidence of disease improvement. Added trial of Symdeko for members 6 years and older. |
| **04/27/2022** | Policy transferred to new template. Removed trial of Symdeko. Clarified dosing. Amended references. |

References:


1. Orkambi [package insert]. Boston, MA: Vertex Pharmaceuticals Inc; 2019.
2. Ren CL, Morgan RL, Oermann C, et al. Cystic Fibrosis Foundation Pulmonary Guidelines. Use of Cystic Fibrosis Transmembrane Conductance Regulator Modulator Therapy in Patients with Cystic Fibrosis. *Ann Am Thorac Soc*. 2018;15(3):271-280. doi:10.1513/AnnalsATS.201707-539OT


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