CareSource

PHARMACY POLICY STATEMENT
Marketplace

DRUG NAME	Orkambi (lumacaftor/ivacaftor)
BILLING CODE	Must use valid NDC
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Orkambi is a combination of ivacaftor, a cystic fibrosis transmembrane conductance regulator (CFTR) potentiator, and lumacaftor originally approved by the FDA in 2015. It is indicated for the treatment of cystic fibrosis (CF) in patients aged 1 year and older who are homozygous for the F508del mutation in the CFTR gene. If the patient's genotype is unknown, an FDA-cleared CF mutation test should be used to detect the presence of the F508del mutation on both alleles of the CFTR gene.

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

Cystic Fibrosis

For **initial** authorization:

1. Member must be 1 year of age or older; AND
2. Member has a diagnosis of cystic fibrosis; AND
3. Medication must be prescribed by or in consultation with a pulmonologist or an infectious disease specialist; 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:** (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), ≥ 14 kg or greater: one packet of granules (each containing lumacaftor 150 mg/ivacaftor 188 mg) taken orally every 12 hours.
   d) Pediatric members age 1 through 2 years ≥ 14 kg or greater: one packet of granules (each containing lumacaftor 150 mg/ivacaftor 188 mg), 9 kg to <14 kg: one packet of granules (each containing lumacaftor 100 mg/ivacaftor 125 mg), 7 kg to <9 kg: one packet of granules (each containing lumacaftor 75 mg/ivacaftor 94 mg) taken orally every 12 hours.

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

For **reauthorization**:

1. Chart notes that show signs and symptoms of improvement, with 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.
09/22/2022	Age requirement expanded to 1 year of age and older. Updated dosing requirement.

References:

1. Orkambi [package insert]. Boston, MA: Vertex Pharmaceuticals Inc; September 2022.
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
3. National Guideline Clearinghouse (NGC). Guideline summary: Cystic fibrosis pulmonary guidelines. Chronic medications for maintenance of lung health. In: National Guideline Clearinghouse (NGC) [Web site]. Rockville (MD): Agency for Healthcare Research and Quality (AHRQ); 2013 Apr 01. Available: https://www.guideline.gov.

Effective date: 04/01/2023
Revised date: 09/22/2022