HAP CareSource™

PHARMACY POLICY STATEMENT
HAP CareSource™ Marketplace

DRUG NAME	Orkambi (lumacaftor/ivacaftor)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Orkambi, 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 (CF) in patients aged 1 year 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 (CF)

For **initial** authorization:

1. Member is at least 1 year 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.
5. **Dosage allowed/Quantity limit:** Per table below. Quantity limit: 112 tablets per 28 days or 56 unit-dose packets per 28 days.

Age	Weight	Dose (every 12 hours)
1 through 2 years	7 kg to <9 kg	1 packet of lumacaftor 75 mg/ivacaftor 94 mg granules
	9 kg to <14 kg	1 packet of lumacaftor 100 mg/ivacaftor 125 mg granules
	≥14 kg	1 packet of lumacaftor 150 mg/ivacaftor 188 mg granules
2 through 5 years	<14 kg	1 packet of lumacaftor 100 mg/ivacaftor 125 mg granules
	≥14 kg	1 packet of lumacaftor 150 mg/ivacaftor 188 mg granules
6 through 11 years	-	2 tablets of lumacaftor 100 mg/ivacaftor 125 mg (lumacaftor 200 mg/ivacaftor 250 mg per dose)

12 years and older	-	2 tablets of lumacaftor 200 mg/ivacaftor 125 mg (lumacaftor 400 mg/ivacaftor 250 mg per dose)

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:

1. 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

HAP 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.
01/27/2025	Added references; increased initial approval length to 6 months.

References:
1. Orkambi [package insert]. Boston, MA: Vertex Pharmaceuticals Inc; 2024.
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. Farrell PM, White TB, Ren CL, et al. Diagnosis of Cystic Fibrosis: Consensus Guidelines from the Cystic Fibrosis Foundation [published correction appears in J Pediatr. 2017 May;184:243]. J Pediatr. 2017;181S:S4-S15.e1. doi:10.1016/j.jpeds.2016.09.064
4. Southern KW, Castellani C, Lammertyn E, et al. Standards of care for CFTR variant-specific therapy (including modulators) for people with cystic fibrosis. J Cyst Fibros. 2023;22(1):17-30. doi:10.1016/j.jcf.2022.10.002
5. 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: 07/01/2025
Revised date:01/27/2025