UnitedHealthcare Community Plan

Clinical Pharmacy Program Guidelines for Orkambi

Program	Prior Authorization
Medication	Orkambi™ (lumacaftor/ivacaftor)
Issue Date	6/2015
Markets in Scope	Arizona, California, Florida- CHIP, Hawaii, Maryland, Nevada, New Jersey, New York, New York EPP, Ohio, Pennsylvania Medicaid, Pennsylvania- CHIP, Rhode Island
Pharmacy and Therapeutics Approval Date	9/2020
Effective Date	11/2020

1. Background:

Orkambi 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 age 2 years 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.

Limitations of Use:
The efficacy and safety of Orkambi have not been established in patients with CF other than those homozygous for the *F508del* mutation.

Members will be required to meet the coverage criteria below.

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2. Coverage Criteria:

A. Initial Authorization

1. **Orkambi** will be approved based upon **all** of the following criteria:

   a. Diagnosis of cystic fibrosis (CF)

   -AND-

   b. Submission of laboratory results confirming that patient is homozygous for the *F508del* mutation in the CFTR gene.

   -AND-

   c. The patient is ≥ 2 years of age

   -AND-

   d. Prescribed by or in consultation with a specialist affiliated with a CF care center

Authorization will be issued for 12 months.

B. Reauthorization

1. **Orkambi** will be approved based on **both** of the following criteria:

   a. Provider attests that the patient has achieved a clinically meaningful response while on Orkambi therapy to **one** of the following:

      (1) Lung function as demonstrated by percent predicted expiratory volume in 1 second ($ppFEV_1$)
      (2) Body mass index (BMI)
      (3) Pulmonary exacerbations
      (4) Quality of life as demonstrated by Cystic Fibrosis Questionnaire-Revised (CFQ-R) respiratory domain score

   -AND-

   b. Prescribed by or in consultation with a specialist affiliated with a CF care center

Authorization will be issued for 12 months.

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3. Additional Clinical Rules:

- Notwithstanding Coverage Criteria, UnitedHealthcare may approve initial and re-authorization based solely on previous claim/medication history, diagnosis codes (ICD-10) and/or claim logic. Use of automated approval and re-approval processes varies by program and/or therapeutic class.
- Supply limits may be in place

4. References:

1. Orkambi [Package Insert]. Boston, MA: Vertex Pharmaceuticals, Inc.; July 2019.

Program	Prior Authorization– Orkambi™ (lumacaftor/ivacaftor)
Change Control	
6/2015	New Program
7/2016	Updated policy template. Aligning with E&I on clinical criteria but changed reauthorization duration from 24 to 12 months.
11/2016	Program updated removing age restriction as label updated for broader pediatric use. Revised prescriber criterion. Updated reference.
11/2017	Annual review. Changed initial authorization from 6 to 12 months.
9/2018	Updated background and criteria for expanded indication in patients as young as 2 years old. Updated reference.
9/2019	Annual review. Updated reference. No change to clinical criteria.
9/2020	Annual review with no changes to coverage criteria. Added Additional Clinical Rules section.

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