UnitedHealthcare Community Plan

Clinical Pharmacy Program Guidelines for Orkambi

Program	Prior Authorization
Medication	Orkambi™ (lumacaftor/ivacaftor)
Issue Date	6/2015
Pharmacy and Therapeutics Approval Date	11/2017
Effective Date	1/2018

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 6 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.

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 ≥ 6 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.

3. References:

1. Orkambi [Package Insert]. Cambridge, MA: Vertex Pharmaceuticals, Inc.; September 2016.

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.