Commercial/Healthcare Exchange PA Criteria

Effective: August 28th, 2018

Prior Authorization: Orkambi

Products Affected: Orkambi (ivacaftor - lumacaftor) oral tablets, Orkambi (ivacaftor - lumacaftor) oral granules

Medication Description:

Orkambi, a combination of lumacaftor and ivacaftor is indicated for the treatment of cystic fibrosis (CF) in patients ≥ 2 years of age who are homozygous for the F508del mutation in the cystic fibrosis transmembrane regulator (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. The efficacy and safety of Orkambi have not been established in patients with CF other than those homozygous for the F508del mutation. Orkambi contains a new chemical entity, lumacaftor, which is a CFTR corrector that increases trafficking of F508del CFTR to the cell surface, and ivacaftor (the same active ingredient contained in Kalydeco [ivacaftor tablets and granules]), a CFTR potentiator that enhances chloride transport of CFTR on the cell surface. The F508del mutation in CFTR causes CF by limiting the amount of CFTR protein that reaches the epithelial cell surface.

Covered Uses: Cystic fibrosis, in patients **homozygous** for the F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene

Exclusion Criteria: N/A

Required Medical Information:

1. Documented diagnosis of homozygous cystic fibrosis for the Phe508del (F508del) mutation in the CFTR gene

Age Restrictions: 2 years of age and older

Prescriber Restrictions: Prescribed by or in consultation with a pulmonologist or a physician who specializes in the treatment of Cystic fibrosis.

Coverage Duration:

Initial: 12 months
Continuation: 3 years

Other Criteria:

Approve Orkambi oral granules if the patient meets the following criteria (A, B, and C):

A. The patient is between 2 and 5 years of age; **AND**
B. The patient has a documented diagnosis of cystic fibrosis; **AND**
C. The patient has been tested using an FDA-cleared CF mutation test which detected the presence of the F508del mutation on both alleles of the CFTR gene

Approve Orkambi oral tablets if the patient meets the following criteria (A, B, and C):

A. The patient is 6 years of age or older; **AND**

B. The patient has a documented diagnosis of cystic fibrosis; **AND**
C. The patient has been tested using an FDA-cleared CF mutation test which detected the presence of the F508del mutation on both alleles of the CFTR gene

References:

1. Orkambi [prescribing information]. Cambridge, MA: Vertex Pharmaceuticals, Inc; August 2018.

Policy Revision history

Rev #	Type of Change	Summary of Change	Sections Affected	Date
1	New Policy Policy update pursuant to label change	Lowered age restriction to 2 years Updated criteria for dose forms indicated for age groups	Age Restrictions and Other Criteria	8/28/18
2	Policy Update	Added continuation coverage duration of 3 years	Coverage Duration	7/1/2019