Effective date: 01/01/2019
Reviewed date: 8/2019, 6/2020, 02/2021, 02/2022, 9/2022, 02/2023 Scope: Medicaid

SPECIALTY GUIDELINE MANAGEMENT

ORKAMBI (lumacaftor/ivacaftor)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met, and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication
Treatment of cystic fibrosis (CF) in patients age 1 years and older who are homozygous for the *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR)* gene. If the patients 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.

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

All other indications are considered experimental/investigational and are not a covered benefit.

II. DOCUMENTATION

The following information is necessary to initiate the prior authorization review: genetic testing report confirming the presence of the appropriate *CFTR* gene mutation.

III. CRITERIA FOR INITIAL APPROVAL

Cystic Fibrosis
Authorization of 6 months may be granted for treatment of cystic fibrosis when all of the following criteria are met:

A. Genetic testing was conducted to detect a mutation in the CFTR gene.
B. The medication is prescribed by or in consultation with a pulmonologist.
B. The member is positive for the *F508del* mutation on both alleles of the CFTR gene.
C. The member is at least 1 year of age.
D. Orkambi will not be used in combination with other ivacaftor containing medications.
E. If requesting granules, member is between 1 year of age through 5 years of age.

IV. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in Section III who are experiencing benefit from therapy as evidenced by disease stability or disease improvement (e.g., improvement in FEV1 from baseline).

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V. QUANTITY LIMIT

Orkambi has a quantity limit of 4 tablets per day, or 2 packets per day.

VI. REFERENCES

1. Orkambi [package insert]. Boston, MA: Vertex Pharmaceuticals Inc.; September 2022.