Reference number(s)
1885-A

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
Orkambi is a combination of lumacaftor and ivacaftor indicated for the treatment of cystic fibrosis (CF) in patients aged 1 year and older who are homozygous for the *F508del* mutation in the cystic fibrosis transmembrane conductance 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.

Limitation of use: The efficacy and safety of Orkambi have 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 medically necessary.

II. DOCUMENTATION

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

III. PRESCRIBER SPECIALTIES

This medication must be prescribed by or in consultation with a pulmonologist.

IV. CRITERIA FOR INITIAL APPROVAL

Cystic Fibrosis
Authorization of 12 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 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 medications containing ivacaftor.

V. CONTINUATION OF THERAPY

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

VI. REFERENCES

1. Orkambi [package insert]. Boston, MA: Vertex Pharmaceuticals Inc.; July 2019.