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 2 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 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 a covered benefit.

II. REQUIRED 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 member is positive for the *F508del* mutation on both alleles of the CFTR gene.
C. The member is at least 2 years of age.
D. Orkambi will not be used in combination with Kalydeco or Symdeko.
E. If requesting granules, member is between 2 through 5 years of age.

IV. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must meet all initial authorization criteria.

V. REFERENCES

1. Orkambi [package insert]. Boston, MA: Vertex Pharmaceuticals Inc.; August 2018.