Reference number(s)
1885-A

Specialty Guideline Management Orkambi

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Orkambi	lumacaftor/ivacaftor

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[1]

Orkambi is indicated for the treatment of cystic fibrosis (CF) in patients aged 1 year and older who are homozygous for the F508del variant in the cystic fibrosis transmembrane conductance regulator (CFTR) gene. If the patient's genotype is unknown, an FDA-cleared CF variant test should be used to detect the presence of the F508del variant 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 variant.

All other indications are considered experimental/investigational and are not medically necessary.

Reference number(s)
1885-A

Documentation

Submission of the following information is necessary to initiate the prior authorization review: For initial requests, genetic testing report confirming the presence of the appropriate CFTR gene variant.

Prescriber Specialties

This medication must be prescribed by or in consultation with a pulmonologist or a prescriber specialized in the treatment of cystic fibrosis.

Coverage Criteria

Cystic Fibrosis[1]

Authorization of 12 months may be granted for treatment of cystic fibrosis when all of the following criteria are met:

- Genetic testing was conducted to detect a variant in the CFTR gene.
- The member is homozygous for the F508del variant (positive for the F508del variant on both alleles) of the CFTR gene.
- The member is at least 1 year of age.

Continuation of Therapy

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the coverage criteria who are experiencing benefit from therapy as evidenced by disease stability or disease improvement (e.g., improvement in forced expiratory volume 1 [FEV1] from baseline).

Other

Orkambi will not be used in combination with another CFTR modulator for the treatment of cystic fibrosis (e.g., Alyftrek, Symdeko).

Reference number(s)
1885-A

Reference

1. Orkambi [package insert]. Boston, MA: Vertex Pharmaceuticals Incorporated; March 2026.