Cystic Fibrosis Agents (Kalydeco and Orkambi)

**BENEFIT APPLICATION**

Benefit determinations are based on the applicable contract language in effect at the time the services were rendered. Exclusions, limitations or exceptions may apply. Benefits may vary based on contract, and individual member benefits must be verified. Wellmark determines medical necessity only if the benefit exists and no contract exclusions are applicable. This policy may not apply to FEP. Benefits are determined by the Federal Employee Program.

**DESCRIPTION**

The intent of the Cystic Fibrosis Agent Policy is to ensure appropriate selection of patients for therapy based on product labeling, clinical guidelines and clinical studies for Kalydeco (ivacaftor) and Orkambi (ivacaftor/lumacaftor).

**FDA-Approved Indications**

Kalydeco is a cystic fibrosis transmembrane conductance regulator (CFTR) potentiator indicated for the treatment of cystic fibrosis (CF) in patients age 2 years and older who have one mutation in the CFTR gene that is responsive to ivacaftor potentiation based on clinical and/or in vitro assay data.

If the patient’s genotype is unknown, an FDA-cleared CF mutation test should be used to detect the presence of CFTR mutation followed by verification with bi-directional sequencing when recommended by the mutation test instructions for use.

Orkambi (lumacaftor/ivacaftor) is approved by the FDA for the treatment of CF in patients age 6 years and older that are homozygous for the F508del mutation in the CFTR gene.

The efficacy and safety of Orkambi have not been established on patients with CF other than those homozygous for the F508del mutation.

**POLICY**

**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.

**CRITERIA FOR INITIAL APPROVAL**

I. **Kalydeco (ivacaftor)** may be considered **medically necessary** for the treatment of cystic fibrosis when all of the following criteria are met:
   - Genetic testing was conducted to detect a mutation in the **CFTR** gene.
• The patient is at least 2 years of age  
• Kalydeco will not be used in combination with Orkambi or Symdeko

Approval will be for lifetime.

II. Orkambi (lumacaftor/ivacaftor) may be considered medically necessary for the treatment of cystic fibrosis when all of the following criteria are met:  
• The patient is at least 6 years of age  
• Genetic testing was conducted to detect a mutation in the CFTR gene  
• The patient is positive for the F508del mutation on both alleles of the CFTR gene  
• The patient will not be using Orkambi in combination with Kalydeco or Symdeko  

Approval will be for lifetime.

III. Orkambi (lumacaftor/ivacaftor) and Kalydeco (ivacaftor) are considered not medically necessary for patients who do not meet the criteria set forth above.

CONTINUATION OF THERAPY

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

Quantity Limits Apply:  
• Kalydeco 56 tablets/28 days  
• Kalydeco Pak 56 packets/28 days  
• Orkambi 112 tablets/28 days

PROCEDURES AND BILLING CODES

To report provider services, use appropriate CPT* codes, Alpha Numeric (HCPCS level 2) codes, Revenue codes, and/or ICD diagnostic codes.  
• No applicable codes

REFERENCES


POLICY HISTORY

Policy #: 05.01.39  
Original Effective Date: May 2012  
Reviewed: January 2018  
Revised: April 2018  
Current Effective Date: April 16, 2018