

PRIOR AUTHORIZATION CRITERIA

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|--|---|-----------------------------|
| BRAND NAME (Generic) | ORKAMBI (lumacaftor/ivacaftor) | |
| Status: CVS Caremark Criteria Type: Initial Prior Authorization | | MDC Ref # 1279-A |

FDA-Approved Indication¹

Orkambi is indicated for the treatment of cystic fibrosis (CF) in patients age 6 years and older who are homozygous for the *F508del* mutation in the *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.

CRITERIA FOR APPROVAL

| | | | |
|---|---|-----|----|
| 1 | Does the patient have a diagnosis of cystic fibrosis? [If no, no further questions.] | Yes | No |
| 2 | Does the patient have the F508del mutation in the cystic fibrosis transmembrane conductance regulator (<i>CFTR</i>) gene? [If no, no further questions.] | Yes | No |
| 3 | Is the patient homozygous for the F508del mutation? [If no, no further questions.] | Yes | No |
| 4 | Will Orkambi be used in combination with Kalydeco? [If yes, no further questions.] | Yes | No |
| 5 | Is the patient 6 years of age or older? | Yes | No |

| Guidelines for Approval | |
|-------------------------------|-------------------|
| Duration of Approval | 12 Months |
| Set 1: Cystic Fibrosis | |
| Yes to question(s) | No to question(s) |
| 1 | 4 |
| 2 | |
| 3 | |
| 5 | |

| Mapping Instructions | | |
|----------------------|--------------------|---------|
| | Yes | No |
| 1. | Go to 2 | Deny |
| 2. | Go to 3 | Deny |
| 3. | Go to 4 | Deny |
| 4. | Deny | Go to 5 |
| 5. | Approve, 12 months | Deny |

RATIONALE

These criteria meet the Medicare Part D definition of a medically accepted indication. This definition includes uses which are approved by the FDA or supported by a citation included, or approved for inclusion, in one of the Medicare approved compendia.

The intent of the criteria is to ensure that patients follow selection elements noted in labeling and/or practice guidelines in order to decrease the potential for inappropriate utilization.

REFERENCE

1. Orkambi [package insert]. Boston, MA: Vertex Pharmaceuticals Inc.; September 2016.

DOCUMENT HISTORY

Written: Specialty Clinical Development (TS) 07/2015
Revised: TS 08/2015 (CMS), IP 01/2016, 07/2016 (CMS), 09/2016 (label update), DK 12/2016, 07/2017 (CMS)
Reviewed: CDPR / KRU 07/2015, DHR 01/2016; GAD 10/2016, LMS 01/2017
External Review: 07/2015, 03/2016, 03/2017