Division: Pharmacy Policy	Subject: Prior Authorization Criteria
Original Development Date: Original Effective Date: Revision Date:	August 3, 2015 April 11, 2016; October 24, 2016; August 10, 2018; November 19, 2019; May 11, 2023

ORKAMBI® (lumacaftor; ivacaftor)

LENGTH OF AUTHORIZATION: 6 months

INITIAL REVIEW CRITERIA:

- Patient must be ≥ 12 months old; AND
- Patient must have a confirmed diagnosis of Cystic Fibrosis; AND
- Patient must be determined to be **homozygous** for the ***F508del*** mutation in the CFTR gene as confirmed by an FDA-approved CF mutation test; AND
- Patients <18 must have undergone a baseline ophthalmic examination to monitor for lens opacities/cataracts.
- Baseline serum transaminases and bilirubin are required prior to therapy.
- Baseline documented percent predicted FEV_1 within the previous 30 days.
- Please note clinical experience in patients with percent predicted FEV_1 ($ppFEV_1$) <40 is limited, and additional monitoring of these patients is recommended during initiation of therapy.

CONTINUATION OF THERAPY

- Disease response as indicated by two or more of the following:
  - Decreased pulmonary exacerbations compared to pretreatment baseline.
  - Improvement or stabilization of lung function (as measured by percent predicted FEV_1) compared to baseline or decrease in the rate of decline of lung function.
  - Weight gain
  - Clinical notes documenting improvement of patient symptoms.
- Patient has not received a lung transplant.
- Patient has not experienced unacceptable toxicity from the drug.
- Submission of liver function tests (every three months) with initial reauthorization is required, then one liver function test annually thereafter.
- Patients ages < 18 should have a follow up ophthalmic examination at least annually.

DOSING & ADMINISTRATION:

- Refer to product labeling at https://www.accessdata.fda.gov/scripts/cder/daf/
- Tablets: lumacaftor 100 mg and ivacaftor 125 mg; lumacaftor 200 mg and ivacaftor 125 mg.
- Oral granules: Unit-dose packets of lumacaftor 100 mg and ivacaftor 125 mg; lumacaftor 150 mg and ivacaftor 188 mg.