Criteria Based Consultation Prescribing Program

CRITERIA FOR DRUG COVERAGE

lumacaftor-ivacaftor (Orkambi®)

**Initial approval criteria:** Non-formulary lumacaftor-ivacaftor (Orkambi®) will be covered on the prescription drug benefit for 12 months when the following criteria are met:

- Prescriber specializes in the treatment of cystic fibrosis
  - AND -
- Patient is at least 2 years of age
  - AND -
- Two copies of delta F508 mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene
  - AND -
- If 6+ years of age: baseline percent predicted FEV₁ (volume of air that can be forced out of lungs in one second after taking a deep breath compared with normal reference values) is at least 30%

**Continued use criteria:** Non-formulary lumacaftor-ivacaftor (Orkambi®) will continue to be covered on the prescription drug benefit for 12 months when the following criteria are met:

- Patient is adherent to therapy regimen as evidenced by refill history and clinic visits.
  - AND -
- AST, ALT, bilirubin, and ophthalmic changes^ are routinely monitored at least annually
  - AND -
- If 6+ years of age: Clinically meaningful response to therapy as evidenced by at least one of the following:
  i. Improved or stabilized percent predicted FEV₁
  ii. Peds and adolescents: BMI increased or stabilized within growth curve percentile
  iii. Adults: Bodyweight increased or stabilized
  iv. Decreased exacerbations and/or hospitalizations (pulmonary related)
  v. Prescriber attests that the patient has a clinically meaningful response to therapy

**Notes:**

- If the patient’s genotype is unknown, an FDA-approved CF mutation test should be used to detect the presence of a CFTR mutation followed by verification with bi-directional sequencing when recommended by the mutation test instructions for use.
- Transaminases (ALT and AST) should be assessed at baseline, every 3 months for one year, and annually thereafter. Increased monitoring may be necessary in patients with a history of elevated ALT, AST, or bilirubin.
- ^ Patients up to 17 years of age: Ophthalmic examination recommended prior to initiation of therapy and at follow-up intervals.
- Strong inducers of CYP3A inducers reduce exposure of ivacaftor, which may diminish its effectiveness; therefore, co-administration is not recommended.