Criteria Based Consultation Prescribing Program
CRITERIA FOR DRUG COVERAGE
ivacaftor (Kalydeco)

Initial approval criteria: Non-formulary ivacaftor (Kalydeco) 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 6 months of age
- AND-
- Patient is NOT homozygous for the F508del mutation in the CFTR gene
- AND-
- At least one of the following mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene:
  - P67L
  - R117C
  - R347H
  - S549R
  - E831X
  - K1060T
  - R1070W
  - S1251N
  - 2789+5G→A
  - R74W
  - G178R
  - R117H
  - R352Q
  - G551S
  - S977F
  - G1069R
  - D1152H
  - D1270N
  - 3849+10kbC→T
  - D110H
  - L206W
  - S549N
  - D579G
  - F1052V
  - R1070Q
  - G1244E
  - G1349D
  - 711+3A→G
  - E56K
- OR-
- Patients with a R117H mutation in the CFTR gene who have clinically significant disease (patients with R117H and the 5T form of the poly-T tract, but not 7T or 9T)

Continued use criteria: Non-formulary ivacaftor (Kalydeco) 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 monitored at least annually
- AND-
- If 6+ years of age: a clinically meaningful response to therapy as evidenced by at least one of the following:
  - Improved or stabilized percent predicted FEV₁
  - Peds and adolescents: BMI increased or stabilized within growth curve percentile
  - Adults: weight increased or stabilized
  - Decreased exacerbations and/or hospitalizations (pulmonary related)
  - 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.