SPECIALTY GUIDELINE MANAGEMENT

KALYDECO (ivacaftor)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication
Kalydeco is a cystic fibrosis transmembrane conductance regulator (CFTR) potentiator indicated for the treatment of cystic fibrosis (CF) in patients age 6 months 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.

All other indications are considered experimental/investigational and are not a covered benefit.

II. 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.

III. CRITERIA FOR INITIAL APPROVAL

Cystic Fibrosis
A 6 month authorization may be granted for treatment of cystic fibrosis when all of the following criteria are met:
A. Genetic testing was conducted to detect a mutation in the *CFTR* gene.
B. The member has one of the following mutations in the *CFTR* gene: A455E, A1067T, D110E, D110H, D579G, D1152H, D1270N, E56K, E193K, F1052V, F1074L, G178R, G551D, G551S, G1069R, G1244E, G1349D, K1060T, L206W, P67L, R74W, R117C, R117H, R347H, R352Q, R1070Q, R1070W, S549N, S549R, S945L, S977F, S1251N, S1255P, 711+3A→G, E831X, 2789+5G→A, 3272-26A→G, 3849+10kbC→T.
C. The member is at least 6 months of age.
D. Kalydeco will not be used in combination with Orkambi or Symdeko.
E. The member does not have the F508del mutation on both alleles of the CFTR gene.
F. If requesting granules, member is between 6 months of age through 5 years of age.

IV. CONTINUATION OF THERAPY

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

V. REFERENCES

1. Kalydeco [package insert]. Boston, MA: Vertex Pharmaceuticals Inc.; August 2018.
2. Mogayzel PJ, Naureckas ET, Robinson KA, et al. Cystic fibrosis pulmonary guidelines. Chronic medications for maintenance of lung health. *Am J Respir Crit Care Med.* 2013;187:680-689.