Commercial/Healthcare Exchange PA Criteria

Effective: April 12th, 2012

Prior Authorization: Kalydeco

Products Affected: Kalydeco Oral Granule Packet, Kalydeco Oral Tablet

Medication Description:

Kalydeco, a cystic fibrosis transmembrane conductance regulator (CFTR) potentiator, is indicated for the treatment of cystic fibrosis (CF) in patients age 12 months and older who have one mutation in the CFTR gene that is responsive to ivacaftor based on clinical and/or in vitro assay data. In patients with unknown genotype, a Food and Drug Administration (FDA)-cleared CF mutation test should be used to detect the presence of the CFTR mutation followed by verification with bidirectional sequencing when recommended by the mutation test instructions for use. Kalydeco is not effective in patients with CF who are homozygous for the phe508del (F508del) mutation in the CFTR.

The CFTR protein is a chloride channel present at the surface of epithelial cells in multiple organs. Kalydeco facilitates increased chloride transport by potentiating the channel-open probability (or gating) of the CFTR protein. More than 1,800 disease-associated changes or mutations have been identified in the CFTR gene. According to the CF patient registry about 47% of patients have two copies of the F508del (Delta F508) mutation; more than 39% of patients with CF have one F508del mutation.

Covered Uses: Cystic fibrosis, in patients with a mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene listed below:

A455E	E56K	G551S	R74W	S549N	2789+5G→A
A1067T	E193K	G1069R	R117C	S549R	3272-26A→G
D110E	E831X	G1244E	R117H	S945L	3849+10kbC→T
D110H	F1052V	G1349D	R347H	S977F	
D1152H	F1074L	K1060T	R352Q	S1251N	
D1270N	G178R	L206W	R1070Q	S1255P	
D579G	G551D	P67L	R1070W	711+3A→G	

Exclusion Criteria: N/A

Required Medical Information:

1. Documented diagnosis
2. Patient has been tested using an FDA cleared test to detect the presence of one CFTR mutation followed by verification with bidirectional sequencing

Age Restrictions: 6 months of age and older for packets. 6 years of age and older for tablets

Prescriber Restrictions: Prescribed by or in consultation with a pulmonologist or a physician who specializes in the treatment of Cystic fibrosis.

Coverage Duration: 3 years

Other Criteria:

Approve Kalydeco if the patient meets the following criteria (A, B, and C):

A. The patient has a documented diagnosis of cystic fibrosis; **AND**
B. The patient has at least **one** mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.

References:

1. Kalydeco® tablets and oral granules [prescribing information]. Boston, MA: Vertex Pharmaceuticals, Inc; March 2019.
2. Kalydeco® tablets and oral granules [prescribing information]. Cambridge, MA: Vertex Pharmaceuticals, Inc; March 2015.

Policy Revision history

Rev #	Type of Change	Summary of Change	Sections Affected	Date
1	New Policy	New Policy	All	05/13/2016
2	Update	Criteria changed to match Updated FDA Label	Required Medical Info Other Criteria	10/03/2018
3	Update	Template Revision (CCI to EH) CCI P&T Review History: 4/12, 10/12, 10/13, 10/14, 11/15, 8/16, 8/17, 7/18 CCI Revision Record: 4/13, 2/15, 8/17, 7/18	All	3/12/19
4	Update	Updated indication/age to match FDA Label	All	3/12/19

5	Update	Added coverage duration for continuation to 3 years	Coverage Duration	7/1/2019
4	Update	Coverage duration updated to 3 years Age Restriction updated: 6 months of age and older for packets. 6 years of age and	Coverage Duration Age Restriction	4/13/2020