Effective date: 1/1/2019
Review: 8/2019, 6/2020, 10/2020, 02/2021, 02/2022, 03/2023, 05/2023, 02/2024, 5/2025 Scope: Medicaid

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 aged 1 month 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 medically necessary.

II. CRITERIA FOR INITIAL APPROVAL

Cystic Fibrosis
Authorization of 6 months may be granted for treatment of cystic fibrosis when all of the following criteria are met:

A. Documentation that genetic testing was conducted to detect a mutation in the *CFTR* gene.
B. The medication is prescribed by or in consultation with a pulmonologist.
C. The member has one of the following mutations in the *CFTR* gene : A120T, A234D, A349V, A455E, A1067T, D110E, D110H, D192G, D579G, D924N, D1152H, D1270N, E56K, E193K, E822K, E831X, F311del, F311L, F508C, F508C;S1251N, F1052V, F1074L, G178E, G178R, G194R, G314E, G551D, G551S, G576A, G970D, G1069R, G1244E, G1249R, G1349D, H939R, H1375P, I148T, I175V, I807M, I1027T, I1139V, K1060T, L206W, L320V, L967S, L997F, L1480P, M152V, M952I, M952T, P67L, Q237E, Q237H, Q359R, Q1291R, R74W, R75Q, R117C, R117G, R117H, R117L, R117P, R170H, R347H, R347L, R352Q, R553Q, R668C, R792G, R933G, R1070Q, R1070W, R1162L, R1283M, S549N, S549R, S589N, S737F, S945L, S977F, S1159F, S1159P, S1251N, S1255P, T338I, T1053I, V232D, V562I, V754M, V1293G, W1282R, Y1014C, Y1032C, 711+3A→G, 2789+5G→A, 3272-26A→G, 3849+10kbC→T.
D. The member is at least 1 months of age.
E. Kalydeco will not be used in combination with other ivacaftor containing medications.
F. The member does not have the F508del mutation on both alleles of the CFTR gene.
G. If requesting granules, member is between 1 months of age to under 6 years of age.

III. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in Section III who are experiencing benefit from therapy as evidenced by disease stability or disease improvement (e.g., improvement in FEV1 from baseline).

IV. QUANTITY LIMIT

Kalydeco has a quantity limit of 2 tablets/packets per day.

FDA-Recommended Dosing			
Age 6 years and older	One 150 mg tablet every 12 hours with fat containing food		
Packets for pediatric patients 1 month to less than 6 years	**Age**	**Body Weight**	**Dosing**
	1 month to less than 2 months	3 kg or greater	One packet (5.8mg) every 12 hours
	2 months to less than 4 months	3 kg or greater	One packet (13.4mg) every 12 hours
	4 months to less than 6 months	5 kg or greater	One packet (25mg) every 12 hours
	6 months to less than 6 years	5 kg to less than 7 kg	One packet (25mg) every 12 hours
		7 kg to less than 14 kg	One packet (50mg) every 12 hours
		14 kg or greater	One packet (75mg) every 12 hours

V. REFERENCES

1. Kalydeco [package insert]. Boston, MA: Vertex Pharmaceuticals Inc.; December 2024.
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.