

# Specialty Guideline Management

## Kalydeco

### Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Kalydeco	ivacaftor

### 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<sup>1</sup>

Kalydeco is indicated for the treatment of cystic fibrosis (CF) in patients aged 1 month and older who have at least one variant in the cystic fibrosis transmembrane conductance regulator (CFTR) gene that is responsive to ivacaftor based on clinical and/or in vitro assay data.

If the patient's genotype is unknown, an FDA-cleared CF variant test should be used to detect the presence of a CFTR variant followed by verification with bi-directional sequencing when recommended by the variant test instructions for use.

All other indications are considered experimental/investigational and are not medically necessary.

### Documentation

Submission of the following information is necessary to initiate the prior authorization review: For initial requests, genetic testing report confirming the presence of the appropriate *CFTR* gene variant.

Reference number(s)
1884-A

## Prescriber Specialties

This medication must be prescribed by or in consultation with a pulmonologist or a prescriber specialized in the treatment of cystic fibrosis.

## Coverage Criteria

### Cystic Fibrosis<sup>1,2</sup>

Authorization of 12 months may be granted for treatment of cystic fibrosis when all the following criteria are met:

- Genetic testing was conducted to detect a variant in the CFTR gene.
- The member has one of the following variants in the CFTR gene: A120T, A234D, A349V, A455E, A1067T, D110E, D110H, D192G, D579G, D924N, D1152H, D1270N, E56K, E193K, E822K, E831X, F311del, F311L, 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.
- The member is at least 1 month of age.

## Continuation of Therapy

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the coverage criteria who are experiencing benefit from therapy as evidenced by disease stability or disease improvement (e.g., improvement in forced expiratory volume 1 [FEV1] from baseline).

## Other

Kalydeco will not be used in combination with another CFTR modulator for the treatment of cystic fibrosis (e.g., Orkambi, Symdeko).

Reference number(s)
1884-A

## References

1. Kalydeco [package insert]. Boston, MA: Vertex Pharmaceuticals Incorporated.; March 2026.
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. doi: 10.1542/peds.2015-1784