# Clinical Policy: Ivacaftor (Kalydeco)

Reference Number: PA.CP.PHAR.210
Effective Date: 01/18
Last Review Date: 07/18

Coding Implications
Revision Log

**Description**
The intent of the criteria is to ensure that patients follow selection elements established by Pennsylvania Health and Wellness® clinical policy for ivacaftor (Kalydeco®).

**FDA Approved Indication(s)**
Kalydeco 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.

If the patient's genotype is unknown, an FDA-cleared 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.

**Policy/Criteria**
It is the policy of Pennsylvania Health and Wellness that Kalydeco is **medically necessary** when the following criteria are met:

**I. Initial Approval Criteria**

**A. Cystic Fibrosis** (must meet all):

1. Age ≥ 12 months;
2. Diagnosis of cystic fibrosis (CF);
3. Presence of one of the *CFTR* gene mutations in Table 3 of the package insert and listed below:
4. Confirmation that a homozygous *F508del* mutation in the *CFTR* gene is not present;
5. Prescribed total daily dose of Kalydeco does not exceed the following:
   a. Age 12 months to < 6 years and < 14 kg: 100mg (granules);
   b. Age 12 months to < 6 years and ≥ 14 kg: 150mg (granules);
   c. Age ≥ 6 years: 300mg (tablets).

**Approval duration: 6 months**

**B. Other diagnoses/indications:** Refer to PA.CP.PMN.53 for Medicaid.

**II. Continued Approval**

**A. Cystic Fibrosis** (must meet all)**:**

1. Currently receiving medication via Pennsylvania Health and Wellness benefit or member has previously met all initial approval criteria or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;
2. Member is responding positively to therapy (e.g.: stable or improved pulmonary function, improved quality of life, reduced hospitalization);
3. Prescribed total daily dose of Kalydeco does not exceed the following:

a. Age 12 months to < 6 years and < 14 kg: 100mg (granules);
b. Age 12 months to < 6 years and ≥ 14 kg: 150mg (granules);
c. Age ≥ 6 years: 300mg (tablets).

**Approval duration: 12 months**

**B. Other diagnoses/indications** (1 or 2):

1. Currently receiving medication via Pennsylvania Health and Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.LTSS.PHAR.01) applies.
   **Approval duration: Duration of request or 6 months (whichever is less);** or

2. Refer to PA.CP.PMN.53 for Medicaid..

**Background**

*Description/Mechanism of Action:*
The CFTR protein is a chloride channel present on the surface of epithelial cells in multiple organs. Ivacaftor facilitates increased chloride transport by potentiating the channel-open probability (or gating) of the CFTR protein.

*Formulations:*
Kalydeco

- Oral tablets: 150 mg ivacaftor
- Oral granules: 50 mg or 75 mg (unit-dose packet) ivacaftor

**Table 3: List of *CFTR* Gene Mutations that Produce CFTR Protein and are Responsive to KALYDECO**

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

Treatment of CF in patients age ≥ 2 years who have one of the above mutations in the *CFTR* gene:

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*If the patient's genotype is unknown, an FDA-cleared 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.*

Limitations of use:

- Kalydeco is not effective in patients with CF who are homozygous for the *F508del* mutation in the *CFTR* gene.

## Appendices

### Appendix A: Abbreviation Key

CF: cystic fibrosis
CFTR: cystic fibrosis transmembrane conductance regulator

### Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

| HCPCS Codes | Description |
|---|---|
| N/A | |

| Reviews, Revisions, and Approvals | Date | Approval Date |
|---|---|---|
| References reviewed and updated. | 02/18 | |

## References

i. Kalydeco Prescribing Information. Boston, MA: Vertex Pharmaceuticals, Inc.; July 2017. Available at https://www.kalydeco.com/. Accessed October 26, 2017.
ii. 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. April 1, 2013; 187(7): 680-689.
iii. Farrell PM, White TB, Ren CL et al. Diagnosis of cystic fibrosis: Consensus guidelines from the Cystic Fibrosis Foundation. J Pediatr 2017;181S:S4-15.