

Clinical Policy: Ivacaftor (Kalydeco)

Reference Number: CP.PHAR.210

Effective Date: 05/16

Last Review Date: 05/17

[Coding Implications](#)
[Revision Log](#)

See [Important Reminder](#) at the end of this policy for important regulatory and legal information.

Description

The intent of the criteria is to ensure that patients follow selection elements established by Centene® clinical policy for ivacaftor (Kalydeco®).

Policy/Criteria

It is the policy of health plans affiliated with Centene Corporation® that Kalydeco is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Cystic Fibrosis (must meet all):

1. Age \geq 2 years;
2. Diagnosis of cystic fibrosis (CF);
3. Presence of one of the following *CFTR* gene mutations: *G551D*, *G1244E*, *G1349D*, *G178R*, *G551S*, *R117H*, *S1251N*, *S1255P*, *S549N*, *S549R*;
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 2 to < 6 years and < 14 kg: 100mg (granules);
 - b. Age 2 to < 6 years and \geq 14 kg: 150mg (granules);
 - c. Age \geq 6 years: 300mg (tablets).

Approval duration: 6 months

B. Other diagnoses/indications: Refer to CP.PHAR.57 - Global Biopharm Policy.

II. Continued Approval

A. Cystic Fibrosis (must meet all):

1. Currently receiving medication via Centene benefit or member has previously met all initial approval criteria;
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 2 to < 6 years and < 14 kg: 100mg (granules);
 - b. Age 2 to < 6 years and \geq 14 kg: 150mg (granules);
 - c. Age \geq 6 years: 300mg (tablets).

Approval duration: 12 months

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

1. Currently receiving medication via Centene benefit and documentation supports positive response to therapy.
Approval duration: Duration of request or 6 months (whichever is less); or
2. Refer to CP.PHAR.57 - Global Biopharm Policy.

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

FDA Approved Indications:

Kalydeco is a CF transmembrane conductance regulator (CFTR) potentiator/oral tablet or granule formulation indicated for:

- Treatment of CF in patients age ≥ 2 years who have one of the following mutations in the *CFTR* gene:
 - *G551D, G1244E, G1349D, G178R, G551S, R117H, S1251N, S1255P, S549N, S549R.*

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
Policy split from CP.PHAR.54 CF Treatments. Evidence of a “significant improvement in FEV1” under continued approval is replaced with “Member continues to respond positively to Kalydeco therapy in one or more of the following areas: pulmonary function, quality of life, pulmonary exacerbations”. Not having increased LFTs is removed as a discontinuation reason. Continuation approval period is extended from 6 to 12 months.	05/16	05/16
Dosing criteria expanded by age. Efficacy statement edited to indicate general positive response to therapy.	05/17	05/17

References

1. Kalydeco Prescribing Information. Boston, MA: Vertex Pharmaceuticals, Inc.; February 2017. Available at http://pi.vrtx.com/files/uspi_ivacaftor.pdf. Accessed May 1, 2017.
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.* April 1, 2013; 187(7): 680-689.

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. “Health Plan” means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan’s affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

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Note: For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.

Note: For Medicare members, to ensure consistency with the Medicare National Coverage Determinations (NCD) and Local Coverage Determinations (LCD), all applicable NCDs, LCDs, and Medicare Coverage Articles should be reviewed prior to applying the criteria set forth in this clinical policy. Refer to the CMS website at <http://www.cms.gov> for additional information.

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