

Clinical Pharmacy Program Guidelines for Kalydeco

Program	Prior Authorization
Medication	Kalydeco™ (ivacaftor)
Markets in Scope	Arizona, California, Hawaii, Maryland, Nevada, New Jersey, New York, New York EPP, Pennsylvania- CHIP, Rhode Island, South Carolina
Issue Date	6/2015
Pharmacy and Therapeutics Approval Date	8/2020
Effective Date	10/2020

1. Background:

Kalydeco (ivacaftor) is a cystic fibrosis transmembrane conductance regulator (CFTR) potentiator indicated for the treatment of cystic fibrosis (CF) in patients age 6 months and older who have one of the following mutations in the CFTR gene that is responsive to ivacaftor based on clinical and/or *in vitro* assay data:

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

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.

Members will be required to meet the coverage criteria below.

2. Coverage Criteria:

<p>A. <u>Initial Authorization</u></p> <p>1. Kalydeco will be approved based upon all of the following criteria:</p> <p style="padding-left: 40px;">a. Diagnosis of cystic fibrosis (CF)</p> <p style="text-align: center;">-AND-</p> <p style="padding-left: 40px;">b. Submission of laboratory results confirming that patient has one of the</p>
--

following mutations in the CFTR gene:

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

-AND-

- c. Prescribed by or in consultation with a specialist affiliated with a CF care center

Authorization will be issued for 12 months.

B. Reauthorization

1. **Kalydeco** will be approved based on **both** of the following criteria:

- a. Provider attests that the patient has achieved a clinically meaningful response while on Kalydeco therapy to **one** of the following:
- (1) Lung function as demonstrated by percent predicted expiratory volume in 1 second (ppFEV₁)
 - (2) Body mass index (BMI)
 - (3) Pulmonary exacerbations
 - (4) Quality of life as demonstrated by Cystic Fibrosis Questionnaire-Revised (CFQ-R) respiratory domain score

-AND-

- b. Prescribed by or in consultation with specialist affiliated with a CF care center

Authorization will be issued for 12 months.

3. Additional Clinical Rules:

- Notwithstanding Coverage Criteria, UnitedHealthcare may approve initial and reauthorization based solely on previous claim/medication history, diagnosis codes

(ICD-10) and/or claim logic. Use of automated approval and re-approval processes varies by program and/or therapeutic class.

- Supply limits may be in place.

4. References:

1. Kalydeco [Package Insert]. Cambridge, MA: Vertex Pharmaceuticals, Inc.; April 2019.

Program	Prior Authorization - Kalydeco (ivacaftor)
Change Control	
6/2015	New Program
7/2016	Updated policy template. Aligning with Employer & Individual on clinical criteria but changed reauthorization duration from 24 to 12 months.
11/2016	Revised prescriber criterion
3/2017	Changed initial authorization duration to 12 months
8/2017	Added 23 additional CFTR mutations based on labeling change
8/2018	Updated table of indicated CFTR mutations in background and criteria. Updated references.
8/2019	Annual review. Updated background and reference.
8/2020	Annual review. No changes to clinical criteria. Added Additional Clinical Rules Section.