Kalydeco® (Ivacaftor)

Indication:

"Kalydeco is a cystic fibrosis transmembrane conductance regulator (CFTR) potentiator indicated for the treatment of cystic fibrosis (CF) in patients age 2 years and older who have one of the following mutations in the CFTR gene: G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, or S549R.
Kalydeco is indicated for the treatment of CF in patients age 2 years and older who have an R117H mutation in the CFTR gene.
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 bidirectional sequencing when recommended by the mutation test instructions for use.
Limitations of Use: Not effective in patients with CF who are homozygous for the F508del mutation in the CFTR gene."[1]

Dosage Form/Strength:

Tablet: 150mg
Granules: Unit-dose packets of 50mg, 75mg

Criteria for Approval: [1]

- Diagnosis of Cystic Fibrosis; **AND**,
- Confirmed G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, or S549R mutation in the Cystic Fibrosis Transmembrane Regulator (CFTR) gene from an FDA-cleared CF mutation test;
  - Verification with bidirectional sequencing when recommended by the mutation test instructions; **OR**
  - If lab results from the patient's CF mutation test are not available, provide documentation describing how the prescriber knows that the patient has a G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, S549R, or R117H mutations; **AND**
- Recipient is 2 years of age or older; **AND**
- Kalydeco is not being used concomitantly with a strong CYP3A inducer; **AND**
- Kalydeco is not being used concomitantly with a strong CYP3A inhibitor without dose adjustment to compensate for the interaction.

Criteria for Denial

- Patient is homozygous for the F508del mutation in the CFTR gene; **OR**
- The patient does not have a confirmed diagnosis of Cystic Fibrosis; **OR**
- The patient is less than 2 years of age; **OR**
- Kalydeco is being used concomitantly with a strong CYP3A inducer; **OR**

- Kalydeco is being used concomitantly with a strong CYP3A inhibitor, without dose adjustment to compensate for the interaction.

Criteria for Reauthorization Approval- After 3 Months of Treatment:

- The patient meets all of the criteria for the initial authorization; **AND**,
- There is no evidence of Kalydeco causing detriment or harm to the patient.

Criteria for Reauthorization Approval- After 9 Months of Treatment:

- The patient meets all of the criteria for the initial authorization; **AND**,
- There is no evidence of Kalydeco causing detriment or harm to the patient; **AND**,
- The patient has experienced one of the following: a slowed rate of clinical decline or disease progression, clinical stabilization, or clinical improvement
    - Documentation must be submitted.

Length of Authorization:

- Initial coverage may be approved for 3 months.
- For re-authorization at treatment month 3:
    - Prescriber must follow-up with the patient (via phone call, email, or office visit) to determine if the medication has been detrimental or harmful to the patient.
    - Treatment month 3 authorizations may then be approved for 6 more months.
- For re-authorization at treatment month 9 (after the initial 3 month approval and the subsequent 6 month approval):
    - Documentation must be submitted showing clinical improvement or lack of disease progression (i.e. disease symptoms have improved, stabilized, or the rate of decline/disease progression have slowed).
    - Documentation must also be submitted showing that the medication is not detrimental to, or harming the patient.
    - Treatment month 9 authorizations may be then approved for 1 year.

Quantity Limit:

- Maximum 2 tablets or granule packets per day

Mechanism of Action:

"Ivacaftor is a potentiator of the CFTR protein. The CFTR protein is a chloride channel present at 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."[1]

References:

[1] Kalydeco [package insert]. Boston, MA. Vertex Pharmaceuticals, Inc. March 2015. http://pi.vrtx.com/files/uspi_ivacaftor.pdf Accessed 5/31/2016.