# Preferred Drug List

# NEW DRUG REVIEW

**Proprietary Name:** Kalydeco™

**Common Name:** Ivacaftor

**PDL Category: Cystic Fibrosis Agents**

| Comparable Products | Preferred Drug List Status |
|---|---|
| N/A | |

## Summary

**Indications and Usage:** Indicated for the treatment of cystic fibrosis (CF) in patients age 6 years and older who have a G551D 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 the G551D mutation. [1]

**Mechanism of Action:** Potentiator of the CFTR protein which improves chloride ion and water transport to various tissues (eg, lung, digestive tract). [1]

**Dosage Forms:** Tablets: 150mg

**Recommended Dosage:** Adults and pediatric patients age 6 years and older: one 150 mg tablet taken orally every 12 hours with fat-containing food. Reduce dose in patients with moderate and severe hepatic impairment. Reduce dose when co-administered with drugs that are moderate or strong CYP3A inhibitors. [1]

**Common Adverse Drug Reactions:** Headache, oropharyngeal pain, upper respiratory tract infection, nasal congestion, abdominal pain, nasopharyngitis, diarrhea, rash, nausea, and dizziness. [1]

**Contraindications:** None. [1]

**Manufacturer:** Vertex Pharmaceuticals, Inc.

**Analysis:** Kalydeco™ is indicated for the treatment of cystic fibrosis (CF) in patients age 6 years and older who have a G551D mutation in the CFTR gene. Kalydeco™ is not effective in patients with CF who are homozygous for the F508del mutation in the CFTR gene and has not been studied in other populations of patients with CF. In the trials used to gain FDA approval, Kalydeco™ was evaluated in two randomized, double-blind, placebo-controlled clinical trials including 213 stable patients with CF who have the G551D mutation in the CFTR gene. Kalydeco™ or placebo was added to the current prescribed CF therapies. The use of inhaled hypertonic saline was not allowed. The primary efficacy endpoint in both studies was improvement in lung function as determined by the mean absolute change from baseline in percent predicted pre-dose $FEV_1$ through 24 weeks of treatment. The mean absolute change in percent predicted $FEV_1$ was 10.6 percentage points in Trial 1 and 12.5 percentage points in Trial 2. It is recommended that Kalydeco™ be added to the Preferred Drug List as a non-preferred drug as it is only indicated to treat a specific subset of patients. The Drug Utilization Review (DUR) Commission is currently developing criteria for the use of Kalydeco™.

**IME Recommendation:**

- ☐ Preferred Drug
- ☐ Recommended Drug
- ☐ Non-Preferred Drug
- ☐ Non-Recommended Drug
- ☒ Non-Preferred Drug with Conditions

1. Kalydeco™ [package insert]. Cambridge, MA: Vertex Pharmaceuticals, Inc.; 2012.