ILLINOIS DEPARTMENT OF Healthcare and Family Services

Kalydeco® (ivacaftor capsules) – Prior Authorization Criteria

Kalydeco® is FDA approved for the treatment of cystic fibrosis (CF) in patients 2 years of age and older with one of the following mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene: G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, S549R, or R117H.[1] The mechanism of action involves potentiation of the epithelial cell CFTR protein resulting in improved regulation of salt and water balance in various tissues including the lungs. Clinical studies have demonstrated a positive impact on forced expiratory volume (FEV1), pulmonary exacerbations, weight gain, and quality of life.[2-7] Kalydeco® is not effective in patients with CF who are homozygous for the F508del mutation in the CFTR gene.[1]

Initial Approval Criteria

An initial approval for a period of 3 months will be granted if the following criteria are met:

1. Client is ≥ 2 years of age.
2. Client has diagnosis of CF with documentation of G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, S549R, or R117H CFTR gene mutation on FDA approved CF mutation test.
3. Prescriber specializes in pulmonology or is from a CF center accredited by the Cystic Fibrosis Foundation.
4. If appropriate, patient is receiving and/or has had adequate trials of the following medications:
    - Dornase alfa
    - Hypertonic saline
    - Inhaled or oral antibiotics
5. Baseline liver function tests (ALT/AST) are provided.
6. Baseline FEV1 in clients ≥ 6 years is provided
7. Goals of therapy are provided.

Renewal Criteria

Additional approvals, beyond the initial 3 month approval, will be granted for 6 months at a time if the following criteria are met:

1. Adherence to Kalydeco® therapy is confirmed.
2. Response to therapy is documented (e.g. improved FEV1, weight gain, decreased exacerbations, etc.).
3. Documentation of continued use of standard therapies previously initiated, if appropriate/tolerated, is provided.
4. Liver functions tests (ALT/AST) are provided with each renewal during the first year of treatment and annually thereafter.

References

1. Kalydeco (ivacaftor) [package insert]. Cambridge, MA: Vertex Pharmaceuticals; March 2015.
2. Flume PA, O'Sullivan BP, Robinson KA, et al. Cystic fibrosis pulmonary guidelines: chronic medications for maintenance of lung health. *Am J Respir Crit Care Med*. 2007;176(10):957-969.
3. Ramsey BW, Davies J, McElvaney NG, et al. A CFTR potentiator in patients with cystic fibrosis and the G551D mutation. *N Engl J Med*. 2011;365(18):1663-1672.
4. Mogayzel PJ, Naureckas ET, Robinson KA, et al. Cystic fibrosis pulmonary guidelines: chronic medications for maintenance of lung function. *Am J Respir Crit Care Med*. 2013;187(7):680-689.
5. Clancy JP, Johnson SG, Yee SW, et al. Clinical pharmacogenetics implementation consortium guidelines for ivacaftor therapy in the context for CFTR genotype. *Clin Pharmacol Ther*. 2014;95(6):592-597. doi: 10.1038/clpt.2014.54.
6. O'Reilly R, Elphick HE. Development, clinical utility, and place of ivacaftor in the treatment of cystic fibrosis. *Drug Des Devel Ther*. 2013;7:929-937. doi: 10.2147/DDDT.S30345.
7. Whiting P, Maiwenn A, Burgers L, et al. Ivacaftor for the treatment of patients with cystic fibrosis and the G551D mutation: a systematic review and cost-effectiveness analysis. *Health Technol Assess*. 2014(18):1-106. doi: 10.3310/hta18180.