Kalydeco® (ivacaftor) – Expanded indication

- On April 30, 2019, Vertex announced the FDA approval of Kalydeco (ivacaftor), for the treatment of cystic fibrosis (CF) in patients age 6 months and older who have one mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene that is responsive to ivacaftor potentiation based on clinical and/or *in vitro* assay data
    - Previously, Kalydeco was approved in patients age 12 months and older.
- CF is caused by a defective or missing CFTR protein resulting from mutations in the CFTR gene. The defective function or absence of CFTR protein results in poor flow of salt and water into and out of the cell in a number of organs. In the lungs, this leads to the buildup of abnormally thick, sticky mucus that can cause chronic lung infections and progressive lung damage in many patients that eventually leads to death.
- The efficacy of Kalydeco in patients aged 6 months to less than 12 months was extrapolated from patients 6 years of age and older with support from population pharmacokinetic analyses showing similar drug exposure levels in adults and children 6 months to less than 12 months of age. Safety of Kalydeco in this population was derived from a cohort of 11 patients in a 24-week, open-label clinical study. The safety profile of patients in this study was similar to that observed in patients 2 years and older.
- The recommended dose of Kalydeco (oral granules) for patients ages 6 months to less than 6 years is weight based:
    - 5 kg to < 7 kg: one 25 mg packet every 12 hours
    - 7 kg to < 14 kg: one 50 mg packet every 12 hours
    - ≥ 14 kg: one 75 mg packet every 12 hours
    - Kalydeco should be taken with fat-containing food (eg, eggs, butter, peanut butter, cheese pizza, whole-milk dairy products)
- Refer to the Kalydeco drug label for additional dosing recommendations in patients ages 6 years and older.