Kalydeco® (ivacaftor) – Expanded indication

- On September 25, 2020, Vertex announced the FDA approval of Kalydeco (ivacaftor), for treatment of cystic fibrosis (CF) in patients age 4 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.
    - Kalydeco was previously approved for this indication in patients 6 months and older.
    - 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.
- The approval of Kalydeco for the expanded indication was based on data from a cohort in the 24-week, Phase 3 open-label, safety cohort (ARRIVAL) consisting of 6 children with CF ages 4 months to less than 6 months who have one of 10 mutations in the *CFTR* gene. This cohort demonstrated a safety profile similar to that observed in older children and adults.
- The recommended oral dose of Kalydeco for the treatment of CF in patients 4 months to less than 6 months is one 25 mg packet every 12 hours (total daily dose of 50 mg/day).
    - Refer to the Kalydeco drug label for dosing for pediatric patients 6 months or older and in adult patients.