Kalydeco® (ivacaftor) – Expanded indication and new dosage strengths

- On May 3, 2023, Vertex Pharmaceuticals announced the FDA approval of Kalydeco (ivacaftor), for the treatment of cystic fibrosis (CF) in patients age 1 month and older who have at least 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 age 4 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.
- In addition to the expanded indication, the FDA approved two new dosage strengths (5.8 mg and 13.4 mg) of Kalydeco oral granules.
    - The oral granules are also available in a 25 mg, 50 mg, and 75 mg strength and Kalydeco is also available as a 150 mg oral tablet.
- The effectiveness of Kalydeco in patients aged 1 month to less than 24 months was extrapolated from patients 6 years of age and older with support from population pharmacokinetic analyses showing that the exposure of ivacaftor in pediatric patients 1 month to less than 24 months of age is within the range of exposure in adults and pediatric patients 6 years of age and older. The safety of Kalydeco for the expanded indication was derived from a cohort of 7 patients aged 1 month to less than 4 months (mean age 1.9 months at baseline).
- The recommended dosage of Kalydeco (oral granules) for pediatric patients age 1 month to less than 2 months, with a body weight 3 kg or greater, is one packet (containing 5.8 mg ivacaftor) every 12 hours. In pediatric patients age 2 month to less than 4 months, with a body weight 3 kg or greater, the recommended dosage is one packet (containing 13.4 mg ivacaftor) every 12 hours.
    - Refer to the Kalydeco drug label for dosing for patients age 4 months and older.