Kalydeco® (ivacaftor) – Expanded indication

- On August 15, 2018, Vertex announced the FDA approval of Kalydeco (ivacaftor) for the treatment of cystic fibrosis (CF) in patients age 12 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 for use in patients ≥ 2 years of age for this indication.
    - 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 efficacy of Kalydeco in patients 12 months to < 24 months was extrapolated from patients ≥ 6 years of age with support from population pharmacokinetic analyses showing similar drug exposure levels in adults and children 12 months to < 24 months of age.
- Safety of Kalydeco in patients aged 12 to < 24 months was derived from a cohort of 19 patients of this age range in a clinical study and administered either 50 mg or 75 mg of Kalydeco granules twice daily. The safety profile of patients in this trial is similar to that observed in patients ≥ 2 years of age.
- The dose of Kalydeco for pediatric patients 12 months to less than 6 years of age is weight based. Each dose should be administered just before or just after fat-containing food.

Body weight (kg)	Dosage Regimen	Total Daily Dose
7 kg to < 14 kg	One 50 mg packet every 12 hours	100 mg/day
≥ 14 kg	One 75 mg packet every 12 hours	150 mg/day

- The recommended dose of Kalydeco for adults and pediatric patients age ≥ 6 years of age is one 150 mg tablet taken orally every 12 hours with fat-containing food.