Orkambi® (lumacaftor/ivacaftor) – Expanded indication and New formulation approval

- On August 7, 2018, Vertex announced the FDA approval of Orkambi (lumacaftor/ivacaftor), for the treatment of cystic fibrosis (CF) in patients age 2 years and older who are homozygous for the *F508del* mutation in the *CFTR* gene.
    - Orkambi was previously approved for use in patients 6 years and older for this same indication.
    - If the patients' genotype is unknown, an FDA-cleared CF mutation test should be used to detect the presence of the *F508del* mutation on both alleles of the *CFTR* gene.
    - The efficacy and safety of Orkambi have not been established in patients with CF other than those homozygous for the *F508del* mutation.
- CF is a systemic, multi-organ, progressive disease that is present from birth. Approximately 1,300 people in the U.S. ages 2 through 5 years are homozygous for the *F508del* mutation, the most common genetic form of the disease.
- The expanded indication approval is based on an open-label safety study in 60 patients that showed treatment with Orkambi was generally safe and well tolerated for 24 weeks, with a safety profile similar to that in patients ages 6 years and older.
- The recommended dose of Orkambi in patients age 2 through 5 years is based on weight as follows:

Patient age	Orkambi dose	Administration notes
2 through 5 years and weighing less than 14 kg	One lumacaftor 100 mg/ivacaftor 125 mg packet of granules every 12 hours.	The entire content of each packet of oral granules should be mixed with 1 teaspoon (5 mL) of soft food or liquid and administered orally with fat-containing food.
2 through 5 years and weighing 14 kg or greater	One lumacaftor 150 mg/ivacaftor 188 mg packet of granules every 12 hours.	

    - Refer to the Orkambi drug label for additional dosing details.
- To support the expanded indication, Vertex plans to launch Orkambi oral granules in two dosage strengths, 100 mg/125 mg and 150 mg/188 mg. Vertex plans to have Orkambi oral granules available for fulfillment within 2 to 4 weeks.
    - Orkambi is currently available as 100 mg/125 mg and 200 mg/125 mg oral tablets.