Orkambi® (lumacaftor/ivacaftor) – Expanded Indication and New Dosage Form

- On September 28, 2016, Vertex announced the FDA approval of Orkambi (lumacaftor/ivacaftor), for the treatment of cystic fibrosis (CF) in patients age 6 years and older who are homozygous for the *F508del* mutation in the *CFTR* gene.
    - Orkambi was previously approved for use in people ages ≥ 12 years with two copies of the *F508del* mutation.
    - If the patient's 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.
- With this new approval, approximately 11,000 people with CF are eligible for treatment with Orkambi in the United States.
- The efficacy of Orkambi in children ages 6 through 11 years is extrapolated from studies of patients ≥ 12 years of age homozygous for the *F508del* mutation in the *CFTR* gene. Pharmacokinetic analyses demonstrated similar drug exposure levels between these age groups.
- The safety profile from an open-label safety study in 58 patients, aged 6 through 11 years with CF who were homozygous for the *F508del-CFTR* mutation, was similar to that observed in previous studies of patients ≥ 12 years of age.
- The recommended dose of Orkambi (lumacaftor/ivacaftor) in patients 6 to 11 years of age is two 100 mg/125 mg tablets taken orally every 12 hours with fat-containing foods (ie, eggs, cheese, nuts).
    - The recommended dose of Orkambi in patients ≥ 12 years of age is two 200 mg/125 mg tablets taken orally every 12 hours with fat-containing foods.
- To support the expanded indication, Vertex plans to launch Orkambi 100 mg/125 mg tablets as soon as possible. Orkambi is currently available as 200 mg/125 mg tablets.

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