Trikafta® (elexacaftor/tezacaftor/ivacaftor; ivacaftor) – Expanded indication, new formulation

- On April 26, 2023, Vertex announced the FDA approval of Trikafta (elexacaftor/tezacaftor/ivacaftor; ivacaftor), for the treatment of cystic fibrosis (CF) **in patients aged 2 years and older** who have at least one *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene or a mutation in the *CFTR* gene that is responsive based on *in vitro* data.
    - Trikafta was previously approved for this indication in patients aged 6 years and older.
    - If the patient's genotype is unknown, an FDA-cleared CF mutation test should be used to confirm the presence of at least one *F508del* mutation or a mutation that is responsive based on *in vitro* data.
- In addition to the expanded indication, the FDA also approved a new oral granule formulation of Trikafta.
    - Trikafta was previously approved as an oral tablet.
- The approval of Trikafta for the expanded indication was based on a 24-week, open-label study in 75 patients ages 2 through 5 years old with CF. The study evaluated the safety and pharmacokinetics of Trikafta.
    - Trikafta was generally well tolerated, with a safety profile consistent with that observed in older age groups, and led to improvements in sweat chloride concentration, a measure of CFTR function, and lung function.
- The recommended dose of Trikafta for the treatment CF in patients aged 2 years to less than 6 years is based on weight.
    - Less than 14 kg: One packet (containing elexacaftor 80 mg/tezacaftor 40 mg/ivacaftor 60 mg) oral granules in the morning and one packet (containing ivacaftor 59.5 mg) oral granules in the evening.
    - 14 kg or more: One packet (containing elexacaftor 100 mg/tezacaftor 50 mg/ivacaftor 75 mg) oral granules in the morning and one packet (containing ivacaftor 75 mg) oral granules in the evening.
- Refer to the Trikafta drug label for dosing for patients aged 6 years and older.