Trikafta® (elexacaftor/tezacaftor/ivacaftor; ivacaftor) – Updated label, boxed warning added

- On December 20, 2024, Vertex announced the **FDA approval of the expanded use of Trikafta (elexacaftor/tezacaftor/ivacaftor; ivacaftor)** for the treatment of people with cystic fibrosis (CF) ages 2 and older who have at least one *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene or a mutation that is responsive to Trikafta based on clinical and/or *in vitro* data.
    - With this approval, 94 additional non-*F508del CFTR* mutations have been added to the Trikafta label. Refer to the drug label for a complete list of responsive mutations.
- In addition to the expanded use approval, the safety information on liver injury and liver failure has been updated from *Warnings and Precautions* to a *Boxed Warning*.
- The label was updated to include data from Trial 5, a randomized, placebo-controlled, double-blind study in 307 patients aged 6 years and older with CF without an *F508del* mutation. Patients were randomized to Trikafta or placebo. The primary endpoint was the absolute change in percent predicted forced expiratory volume in 1 second ($ppFEV_1$) from baseline through week 24.
    - The treatment difference in absolute change in $ppFEV_1$ from baseline through week 24 was 9.2 percentage points (95% CI: 7.2, 11.3; $p < 0.0001$).