

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

- On April 26, 2023, <u>Vertex announced</u> the FDA approval of <u>Trikafta (elexacaftor/tezacaftor/ivacaftor; ivacaftor)</u>, 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.



At Optum, we help create a healthier world, one insight, one connection, one person at a time. All Optum trademarks and logos are owned by Optum, Inc., in the U.S. and other jurisdictions. All other trademarks are the property of their respective owners. This document contains information that is considered proprietary to Optum Rx and should not be reproduced without the express written consent of Optum Rx. RxNews® is published by the Optum Rx Clinical Services Department.