Generic Name:
Elexacaftor, ivacaftor, tezacaftor
Trade Name:
Trikafta
Company:
Vertex Pharmaceuticals
Notes:

FDA has approved elexacaftor, ivacaftor, and tezacaftor, the first triple-combination therapy to treat patients ages 12 years and older with at least one F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene, which is estimated to represent 90% of the cystic fibrosis population.

Currently available therapies that target the defective protein are treatment options for some patients with cystic fibrosis, but many patients have mutations that are ineligible for treatment.

In clinical trials, serious adverse drug reactions that occurred more frequently in patients receiving the three-drug combination compared with placebo were rash and influenza.

The most common adverse drug reactions included headaches, upper respiratory tract infections, abdominal pains, diarrhea, rashes, increased liver enzymes, nasal congestion, increased blood creatine phosphokinase, rhinorrhea, rhinitis, influenza, sinusitis, and increased blood bilirubin.

The prescribing information includes warnings related to elevated liver function tests (transaminases and bilirubin), use at the same time with other products that are inducers or inhibitors of CYP3A, and the risk of cataracts. Patients and their caregivers should speak with a health care professional about these risks and any medication they take before starting treatment.

Clinicians should confirm the presence of at least one F508del mutation with use of an FDA-cleared genotyping assay before beginning treatment.

The agent's safety and effectiveness in patients with cystic fibrosis younger than 12 years have not been established.

Medication Monitor Categories:
New Drug Approvals