Generic Name:
Tezacaftor/ivacaftor
Trade Name:
Symdeko
Company:
Vertex Pharmaceuticals
Notes:

FDA expanded the indication of tezacaftor/ivacaftor tablets for treatment of pediatric patients aged 6 years and older with cystic fibrosis who have certain genetic mutations. Last year, FDA approved tezacaftor/ivacaftor to treat patients aged 12 and older who had the same specific genetic mutations.

Tezacaftor/ivacaftor is used to treat patients who have two copies of the most common type of mutation?the F508del mutation?or who have at least one of the mutations in the CFTR gene that is responsive to the active ingredients, based on in vitro data and/or clinical evidence. Patients with cystic fibrosis and their caregivers should speak with a health professional and have tests performed to understand which gene mutations patients have and whether tezacaftor/ivacaftor is likely to work for them.

Efficacy for patients with cystic fibrosis aged 12 years and older was evaluated in three Phase III, double-blind, placebo-controlled trials that demonstrated improvements in lung function and other key measures of the disease, including a reduction in exacerbations. Efficacy in patients aged 6 to 12 was extrapolated from patients aged 12 years and older, with additional support from data in patients aged 6 to 12 years.

Safety of the agent to treat patients aged 6 years to younger than 12 years was supported by data from a study that included a 24-week, open-label treatment period with 70 participants.

The medication should always be taken with food that contains fat and never in combination with certain antibiotics, seizure medicines, St. John?s wort, or food containing grapefruit or Seville oranges, as indicated on the label. The prescribing information includes warnings for elevated liver enzymes, for those who use inducers for the CYP3A liver enzyme, and for the risk of cataracts in pediatric patients.

The most common adverse effects include headache, nausea, sinus congestion, and dizziness.

Safety and efficacy in patients with cystic fibrosis who are younger than 6 years old have not been studied.

Medication Monitor Categories:
Supplemental Approvals