FDA expands cystic fibrosis treatment approval to children ages 6 to 12

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(HealthDay)—The indication for a cystic fibrosis treatment, Symdeko (tezacaftor/ivacaftor) tablets, has been expanded to treat children ages 6 years and older with cystic fibrosis and certain genetic mutations, the U.S. Food and Drug Administration announced today.

Symdeko is approved to treat patients with two copies of the *F508del* mutation, the most common type of mutation, and patients who have at least one of the mutations in the *CFTR* gene that is responsive to the active ingredients in Symdeko. The FDA approved Symdeko last year for treatment in children ages 12 years and older with the same genetic mutations. Dosage in children ages 6 to 12 years weighing less than 30 kg is one tablet of 50 mg tezacaftor and 75 mg ivacaftor in the morning and one tablet of 75 mg ivacaftor approximately 12 hours later. In patients aged 12 years and older or who weigh 30 kg or more, dosage is one tablet containing 100 mg tezacaftor and 150 mg ivacaftor and a tablet containing 150 mg ivacaftor 12 hours later.

Symdeko's approval was based on three phase 3, double-blind, placebo-controlled trials that showed improvements in lung function and a reduction in exacerbations. The expanded indication approval to children ages 6 to 12 years was based on data from patients aged 12 years and older and additional support from data on patients aged 6 to 12 years. Symdeko's safety in children ages 6 to 12 years was demonstrated in a 24-week, open-label study of 70 cystic fibrosis patients aged 6 to 12 years.

Symdeko's labeling indicates that it should always be taken with food containing fat and should never be combined with certain antibiotics, seizure medications, St. John's wort, or food containing grapefruit or Seville oranges. The prescribing information includes warnings of elevated transaminases and the risk for cataracts in children taking Symdeko and warns against taking Symdeko simultaneously with CYP3A inducers. The most commonly reported side effects were headache, nausea, sinus congestion, and dizziness.

Approval was granted to Vertex Pharmaceuticals.

More information: More Information