FDA approves new drug for most common form of cystic fibrosis

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(HealthDay)—A new drug to treat most cystic fibrosis patients has been approved by the U.S. Food and Drug Administration.

Trikafta (elexacaftor/ivacaftor/tezacaftor) is the first triple combination therapy available to treat patients with the most common cystic fibrosis mutation. Its list price is $311,000 a year, same as one of the maker's earlier treatments for the genetic disease.

Trikafta is approved for patients aged 12 and older who have at least one F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene. This includes about 90% of cystic fibrosis patients, or about 27,000 people in the United States, according to the FDA.

There are about 2,000 known mutations of the CFTR gene, but the F508del mutation is the most common. Cystic fibrosis is the result of a defective protein caused by the gene mutation.

Current drugs that target the defective protein can be used to treat some patients, but many patients have mutations that don't respond to those treatments, the FDA said.

Trikafta—from Vertex Pharmaceuticals, Inc.—is a combo of three drugs. It helps the defective protein made by the CFTR mutation function more effectively.

Trikafta's approval is based on results of two clinical trials involving 510 patients.

The trials assessed a measure of lung function called percent predicted forced expiratory volume in one second (ppFEV1). It measures how much air a person can exhale during a forced breath.

In one trial, average ppFEV1 among patients who took Trikafta improved 13.8% compared to those who took a placebo. In the second trial, the drug increased average ppFEV1 by 10% compared to the drugs tezacaftor/ivacaftor.

Acting FDA Commissioner Dr. Ned Sharpless said the approval was an outgrowth of efforts to speed development of new therapies for complex diseases.

Trikafta's approval makes a new "treatment available to most cystic fibrosis patients, including adolescents, who previously had no options and giving others in the cystic fibrosis community access to an additional effective therapy," he said in an agency news release.

Cystic fibrosis is a progressive, life-threatening disease. It causes formation of thick mucus that builds up in the lungs, digestive tract and other parts of the body, resulting in severe respiratory and digestive problems and other complications, such as infections and diabetes.

More information: The American Lung

Association has more on cystic fibrosis.

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