MedicalXpress

Kalydeco approval widened for more types of cystic fibrosis

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(HealthDay)—The U.S. Food and Drug Administration says it has expanded approval for the cystic fibrosis drug Kalydeco (ivacaftor) to include 33 mutations of the disease, up from the previous 10 mutations.

In a media release, the agency said its decision was based on results of laboratory testing and previous clinical trials.

"Many rare cystic fibrosis mutations have such small patient populations that clinical trial studies are not feasible," said Dr. Janet Woodcock, director of the agency's Center for Drug Evaluation and Research. "This challenge led us to using an alternative approach based on precision medicine, which made it possible to identify certain gene mutations that are likely to respond to Kalydeco."

People with cystic fibrosis—roughly 30,000 of them in the United States—have a defective gene that causes mucus to become sticky and thick. Secretions gather in the lungs and digestive tract, leading to severe problems with breathing and digestion, and infections, the FDA said.

Common side effects of the drug include headache, upper respiratory-tract infection, stomach pain, diarrhea, rash, nausea and dizziness. St. John's Wort and similar drugs and supplements could diminish Kalydeco's effectiveness and should be avoided, the FDA said.

Kalydeco is produced by Boston-based Vertex Pharmaceuticals.

More information: The FDA has more about this approval.