MedicalXpress

Two drugs offer hope for fatal lung disease

May 18 2014, by Dennis Thompson, Healthday Reporter

Doctors have had little to offer patients with idiopathic pulmonary fibrosis.

(HealthDay)—A pair of drugs offers new hope to patients with a progressive, fatal disease that robs their breath by scarring the lungs, according to clinical trial results.

Both drugs, pirfenidone and nintedanib, appear to slow the advance of idiopathic pulmonary fibrosis, or IPF, an incurable and previously untreatable disease that causes tissue deep in the lungs to stiffen and scar.

Patients with IPF have a three-year survival rate of 50 percent, worse than most forms of cancer, said Dr. Gary Hunninghake, a lung disease specialist at Brigham and Women's Hospital in Boston.

Few live longer than five years past diagnosis, and the only way to save

their lives is to replace their lungs.

"The long and short is, this is a pretty big deal in the field of IPF," Hunninghake said. "It isn't curing the disease. This certainly isn't the end of therapy for these patients. But the idea there is something we can do besides referring them to lung transplant is a pretty big deal."

Results from the two drug trials are published in the May 18 online issue of the *New England Journal of Medicine*. The issue also reports on a third IPF drug called acetylcysteine that proved ineffective in a clinical trial.

More than 100,000 Americans suffer from IPF, said Dr. David Lederer, co-author of the pirfenidone study. Most are between 50 and 70 years old. The disease starts with shortness of breath or a dry, hacking cough, but soon robs the person's body of the oxygen needed to move about or properly function.

Doctors don't know what causes IPF, although they suspect that smoking, genetics, certain viral infections or acid reflux could play a role in damaging the lungs, according to the U.S. National Institutes of Health.

Of the two drugs, pirfenidone offers the most cause for hope among physicians and patients, said Dr. Gregory Cosgrove, chief medical officer for the Pulmonary Fibrosis Foundation.

Pirfenidone is approved for IPF treatment in other countries, but until now the U.S. Food and Drug Administration has denied approval based on mixed clinical trial results, Hunninghake said.

In this latest trial, doctors treated 555 IPF patients for one year with either pirfenidone or an inactive placebo. Pirfenidone produced a

significant reduction in the rate of lung function decline, and improved patients' walking distance.

"This drug very clearly slowed down progression of the disease as measured by lung function, and seems to have an effect on mortality as well," said Lederer, co-director of the interstitial lung disease program at Columbia University Medical Center in New York City.

Researchers don't completely understand how pirfenidone works against IPF, Lederer said, but the drug seems to inhibit an important "growth factor" protein in the development of fibrosis. It also appears to have anti-inflammatory properties.

A second set of clinical trials tested the drug nintedanib against a placebo for one year in over 1,050 IPF patients.

Nintedanib also significantly reduced the rate of lung function decline, improving patients' ability to breathe. The drug specifically targets the "growth factor" proteins that cause lung tissue to stiffen and scar.

Gastrointestinal problems including diarrhea were the most common side effects of both medications, researchers reported.

The matter of approval now rests with the FDA. "Both of these trials were designed more than likely to get FDA approval after their publication," Hunninghake said.

Overall, the results of these trials will provide some much-needed encouragement to IPF patients. "It is an optimistic time for patients with fibrosis," Cosgrove said.

The disease has been difficult to crack because animal models of lung fibrosis don't translate well to humans, he said. But the rapid advance of

DNA sequencing has given researchers another avenue to understand how fibrosis works.

"It's been frustrating when we have not identified an effective therapy over the past 10 to 15 years," Cosgrove said. "But that degree of frustration has prompted the IPF community to really come together to support participation in clinical trials, and those trials have provided a foundation for these new advances."

More information: For more information on idiopathic pulmonary fibrosis, visit the U.S. National Institutes of Health.