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Novel approach shows promise for cystic fibrosis

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An investigational drug targeting a defective protein that causes cystic fibrosis has been shown to improve lung function in a small study of CF patients, according to findings published Nov. 18, 2010, in the *New England Journal of Medicine*. The investigational drug, VX-770, appeared to improve function of what is known as CFTR--the faulty protein responsible for CF. It is among the first compounds being developed for CF that specifically targets the root cause of cystic fibrosis.

Patients who took VX-770 for 28 days showed improvements in several key indicators of cystic fibrosis, including lung function, nasal potential difference measurements and sweat chloride levels. Excessive sweat chloride is a key clinical indicator of CF. The sweat test is the traditional diagnostic test for CF.

"Patients with CF have a defective protein in chloride channels in lung cells that, in effect, causes a door to shut too tightly, ultimately leading to severe infections in the lung" said Steven M. Rowe, M.D., M.S.P.H, assistant professor of medicine at the University of Alabama at Birmingham and lead investigator at the UAB site, one of 16 study sites nationwide for this VX-770 trial. "The data suggest that the drug seems to improve the function of the protein, so that the door opens and closes more properly." UAB also served as the central coordinating center for nasal potential difference testing, an important outcome measure in the trial.

VX-770 was developed by Vertex Pharmaceuticals, in conjunction with the Cystic Fibrosis Foundation, using a cutting-edge technology known as high-throughput screening, an advanced technique for drug discovery. UAB's Gregory Fleming James Cystic Fibrosis Research Center was the first site in the nation to clinically administer VX-770 to CF patients in the trial in May 2007.

Investigators studied 39 patients with CF in the trial who had a specific mutation in the gene known to cause CF. The primary objective of the trial was to demonstrate the safety and tolerability of VX-770. All patients in the trial completed use of the study drug, and adverse events were similar to placebo.

"Nearly a decade ago, the CF Foundation recognized the need to develop new therapies that address the underlying cause of CF and not just the symptoms of the disease. We are encouraged by the data from this Phase 2 trial and see the trial as a milestone in our efforts to discover and develop new treatment options for this disease," said Robert J. Beall, Ph.D., President and CEO of the Cystic Fibrosis Foundation.

VX-770 is currently in Phase 3 clinical trials for cystic fibrosis, and, pending data from these studies, Vertex anticipates submitting a new drug application to the FDA in the second half of 2011.

CF is a fatal genetic disease that affects about 30,000 people in the United States and 70,000 worldwide. It is caused by a genetic mutation that causes life-threatening lung infections and premature death. Ten million Americans are symptomless carriers of a defective CF gene.

Provided by University of Alabama at Birmingham