Study shows new medication effectively treats underlying cause of cystic fibrosis

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A new study has confirmed that the drug, ivacaftor (VX-770), significantly improves lung function in some people with cystic fibrosis (CF). The results of the phase III clinical trial study, "A CFTR Potentiator in Patients with Cystic Fibrosis and the G551D Mutation," led by Bonnie W. Ramsey, MD of Seattle Children's Research Institute and the University of Washington, were published today in the *New England Journal of Medicine*.

Ivacaftor, also known as VX-770, was developed by Vertex Pharmaceuticals with financial support from the Cystic Fibrosis Foundation. The oral medicine targets the defective protein produced by the gene mutation called G551D that causes CF. Researchers found that patients carrying G551D – approximately four per cent of all CF patients – who were treated with VX-770 showed a 17 per cent relative improvement in lung function that was sustained over the course of 48 weeks.

Additionally, patients with G551D treated with VX-770 showed improvements in other areas critically important to the health of people with CF. Study participants experienced significant reductions in sweat chloride levels indicating an improvement in the body's ability to carry salt in and out of cells – a process which when defective leads to CF. They also experienced decreased respiratory distress symptoms and improved weight gain. Those who received VX-770 gained on average seven pounds compared to those in the placebo group who gained approximately one pound. This is significant because many people with

CF have difficulty gaining and maintaining weight due to reduced lung function and chronic infection.

"Our study shows that we are now able to improve the quality of life for cystic fibrosis patients with the G551D mutation with the administration of VX-770," said Dr. Ramsey, director of the Center for Clinical and Translational Research at Seattle Children's Research Institute and endowed chair in Cystic Fibrosis (CF) in the Department of Pediatrics at the University of Washington School of Medicine.

Dr. Ramsey and co-investigators evaluated lung function in patients 12 years or older who carry at least one copy of the G551D mutation. The study included 161 patients at multiple healthcare centers who received at least one dose of VX-770 or placebo. The study is the third and final in a series designed to assess VX-770's effectiveness and safety before it is approved for public use.

Approximately 30,000 children and adults in the United States and 70,000 people worldwide have CF. The disease is caused by a defective gene that affects many parts of the body, but is especially harmful to the lungs and pancreas. The gene mutation causes very thick, sticky mucus to clog the lungs and often cause life-threatening infections. In the 1950s, few children with CF lived beyond age five. Today, advances in research and medical treatments have made it possible for people with CF to live well into their 30s, 40s and beyond.

Provided by Seattle Children's

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