

Combination Therapy Helps Cystic Fibrosis Patients

Molecular manipulation of faulty ion channel reduces exacerbations and loss of lung function

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Denver — Researchers at National Jewish Health and around the world report in the *New England Journal of Medicine* that a novel combination of medications improves lung function and reduces disease exacerbations in [cystic fibrosis](#) patients with the most common form of the disease. The medications also caused fewer negative side effects than existing, approved medications.



“Fewer exacerbations means better lung function for a longer time,” said [Jennifer Taylor-Cousar, MD, MSCS](#), Associate Professor of Medicine at National Jewish Health and co-lead investigator/first author of the new report. “We know that lung function correlates with survival, so that is great news for patients with cystic fibrosis and the doctors who care for them.”

Cystic fibrosis results from a genetic mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene, which produces a faulty ion channel that prevents chloride ions from exiting cells in the lungs and other organs. With chloride unable to exit cells, thick mucus builds up in the lungs and gastrointestinal organs. The thick mucus provides a favorable environment for bacterial and other infections to develop. Patients suffer repeated infections of the lungs, which result in lung damage over time and eventually death.

In recent years, several new medications, which interact directly with the faulty CFTR ion channel, have been developed. Tezacaftor, an investigational medication, corrects defects in the ion channel that keep it from moving from the cell’s interior to its outer surface. Ivacaftor, already approved for treatment of certain forms of cystic fibrosis, helps keep the faulty ion channel on the cell surface open so chloride and other ions can exit the cell.

The U.S. Food and Drug Administration (FDA) has already approved a similar combination of Ivacaftor and Lumacaftor for patients who have two copies of the most common CFTR mutation, F508del. However, that combination has a number of drug-drug interactions and negative side effects that prevent some people from using it.

In the current study, Dr. Taylor-Cousar and colleagues from 91 study sites in the United States, Canada and Europe, evaluated 475 patients with two copies of 508del who took the ivacaftor-tezacaftor combination or a placebo for 24 weeks.

Those taking the placebo experienced a 0.6 percent decline in lung function (forced expiratory volume in one second, or FEV₁) compared to a 3.4 percent improvement for those taking the experimental combination. Those taking the experimental combination experienced pulmonary exacerbations requiring hospitalization or intravenous antibiotics 35 percent less often than those taking the placebo. Patients taking the ivacaftor-tezacaftor combination also experienced fewer respiratory adverse events, including respiratory infections, cough, headache and fatigue, than did

those taking placebo.

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National Jewish Health is the leading respiratory hospital in the nation. Founded 124 years ago as a nonprofit hospital, National Jewish Health today is the only facility in the world dedicated exclusively to groundbreaking medical research and treatment of patients with respiratory, cardiac, immune and related disorders. Patients and families come to National Jewish Health from around the world to receive cutting-edge, comprehensive, coordinated care. To learn more, visit the media resources page.

Media Contacts

Our team is available to arrange interviews, discuss events and story ideas.

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