



Cystic fibrosis treatment has generally been focused on easing symptoms, but a combination of drugs used in a recent study prevented many of the pulmonary exacerbations that lead to patients being hospitalized.

Researchers gave patients a combination of Ivacaftor, used primarily for patients with a specific genetic mutation that causes cystic fibrosis, and Lumacaftor, an experimental drug that addresses another mutation which a high percentage of patients have. The success of the study is being considered groundbreaking because it addresses that disease instead of symptoms.

"While significant progress has been made with supportive therapies for cystic fibrosis, developing treatments that address the underlying genetic cause has been a challenge," said Susanna McColley, Associate Director of the Cystic Fibrosis Center at Lurie Children's Hospital of Chicago, in a press release. "These groundbreaking findings will benefit around 15,000 patients in U.S. alone."

Cystic fibrosis causes the body to make thick mucus, slowly preventing the lungs and pancreas from doing their jobs. The disease is caused by a genetic mutation. The goal of researchers has been to find treatments which can prevent this mutation, and both drugs used in the study have been effective on specific groups of patients.

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