

Cystic Fibrosis Research News

Title:

Recovery of lung function following a pulmonary exacerbation in patients with cystic fibrosis and the G551D-CFTR mutation treated with ivacaftor

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What was your research question?

Episodes of worsening of symptoms in the lungs (pulmonary exacerbations) in patients with cystic fibrosis (CF) often result in loss of lung function. In some cases, patients recover the lost lung function after treatment, but in other cases patients do not recover that lost function. We wanted to know if ivacaftor, a CFTR modulator that treats the basic defect in CF, would improve the chance of recovery of lung function after a pulmonary exacerbation.

Why is this important?

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Pulmonary exacerbations are important events in the lives of persons with CF, both because they interfere with patients' well-being and because most patients who have an exacerbation lose lung function that they will never recover. It is possible that improvement in airway clearance could make the treatment of a pulmonary exacerbation more effective.

What did you do?

We analyzed data collected from the ivacaftor placebo-controlled clinical trials and assessed lung function before and after a pulmonary exacerbation. We looked at both the short-term (2-8 weeks after treatment of the exacerbation) and long-term (end of the study) impact on recovery of lung function. We considered full recovery as achieving 100% of lung function measured just before the exacerbation.

What did you find?

Fewer patients on ivacaftor experienced an exacerbation when compared to those on placebo. However, there was no difference between the groups in the proportion of patients with full recovery in the short or long term. Remarkably, only about half of patients achieve full recovery of lung function after treatment of an exacerbation.

What does this mean and reasons for caution?

This suggests that treatment of the underlying defect in CF does not improve the chance of recovery of lung function that is lost due to pulmonary exacerbations, so it is best to try to avoid these important events.

What's next?

We will continue to do studies looking at best treatments for pulmonary exacerbations, and will also focus on methods to prevent these events.

Original manuscript citation in PubMed

<https://www.ncbi.nlm.nih.gov/pubmed/?term=Recovery+of+lung+function+following+a+pulmonary+exacerbation+in+patients+with+cystic+fibrosis+and+the+G551D-CFTR+mutation+treated+with+ivacaftor>