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Cystic Fibrosis Research News

Title:

RATIONALIZING ENDPOINTS FOR PROSPECTIVE STUDIES OF PULMONARY EXACERBATION TREATMENT RESPONSE IN CYSTIC FIBROSIS

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What was your research question?

For people with cystic fibrosis (CF), we wanted to understand the best ways to measure how an individual's health improves when they are treated for an increase of infection in their lungs (pulmonary exacerbation), and how many participants we would need to take part in a study with medication (clinical trial) to be able to show how the results of exacerbation treatments are different.

Why is this important?

Pulmonary exacerbations are important events in the lives of people with CF, both because they interfere with individuals' well-being and because most individuals who have an exacerbation lose lung function that they will never recover. Although exacerbations are common, treatments vary widely and very little is known about which treatments provide better results. Our future goal is to compare different exacerbation treatments for their safety and ability to provide benefit. To do this, we need to understand how best to measure recovery from exacerbation, and how many individuals need to be studied to prove that treatments are different.

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What did you do?

Starting with results from the recently completed STOP (Standardized Treatment of Pulmonary Exacerbation) study, we measured how lung function changed when people with CF were treated with antibiotics for exacerbation using several different ways of measuring change. We also studied how lung function changes were or were not related to changes in the way that individuals felt as they received exacerbation treatments. We then studied how many participants would be needed in clinical studies comparing two different exacerbation treatments if the different ways of measuring response to treatment were used.

What did you find?

We found that comparing two exacerbation treatments by studying how individuals reported they felt after treatment required the fewest numbers of participants. In contrast, we found that the different ways of measuring changes in lung function were similar and all required more participants to study in clinical trials. We also found that although most participants who reported feeling better after treatment also had better lung function, this was not always the case: some felt better but their lung function hadn't improved and some had improved lung function but didn't feel better.

What does this mean and reasons for caution?

Our results suggest that we will need to run very large clinical studies, including several hundreds to more than a thousand participants, to be able to reliably compare different pulmonary exacerbation treatments. Our results also suggest that we should study both how lung function changes and how individuals report they feel when treated for exacerbation if we want to best understand how exacerbation treatments benefit individuals with CF. Because the STOP study was only conducted at 12 CF care centers in the US, our results may not apply to all people with CF who might benefit from better exacerbation care.

What's next?

Results from this study have been used to help design a large research study at multiple CF care centers that compares how individuals with CF respond when treated with antibiotics for different amounts of time.

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