



# Cystic Fibrosis Research News

## Title:

Safety and Efficacy of Lenabasum in a Phase 2 Randomized, Placebo-Controlled Trial in Adults with Cystic Fibrosis

## Authors:

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## What was your research question?

In this study, we wanted to learn whether lenabasum, an investigational medicine that decreases inflammation, is safe in people who have cystic fibrosis (CF). Reducing inflammation may help decrease lung damage.

## Why is this important?

Currently there is a need for new drugs that treat inflammation in the lungs of people with CF. Despite new drugs like CFTR (cystic fibrosis transmembrane conductance regulator) modulators, people with CF still get lung damage.

Many anti-inflammatory drugs completely turn off the body's immune system. This could increase a person's chance to get other or worse infections. Lenabasum does not completely turn off the body's immune system; lenabasum turns on the endocannabinoid system, which decreases inflammation and reduces the formation of scar tissue. Lenabasum does this by only binding to a receptor called CB2. The idea is lenabasum will reduce inflammation enough to minimise lung damage.

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## What did you do?

We tested the safety and tolerability of different strengths of lenabasum in adults with CF. To see if the lenabasum was safe, we collected reports on side effects, vital signs, and other tests. We also measured how often people in the study stopped the drug, and we looked at lung function, inflammation molecules in the blood and sputum, and how people answered surveys.

This Phase 2a study was a double-blind, randomized, placebo-controlled study, meaning neither patients nor researchers knew who received a placebo (a dummy drug with no active medication) and who received lenabasum. Eighty-five people with CF, who live in the US or Europe, were in the study for 4 months.

## What did you find?

In this study, we found that lenabasum was generally safe and well-tolerated by people with CF. No deaths occurred, and no serious/severe side effects were thought to be related to lenabasum. Most side effects were mild to moderate. The most common side effects included pulmonary exacerbation (a flare-up of symptoms), mild blood-stained mucus, dry mouth, and upper airway infection. Three people who took lenabasum and one person who took placebo stopped the study because of a side effect.

We saw people who took lenabasum tended to have fewer pulmonary exacerbations than the placebo group. Markers of inflammation in the sputum were also reduced in the lenabasum group compared to placebo. These results are encouraging and support further study of lenabasum.

## What does this mean and reasons for caution?

We were able to answer our main research question by showing that lenabasum is safe and well-tolerated in adults with CF. Additionally, we saw initial signs that people who took lenabasum had fewer pulmonary exacerbations. The results of this clinical trial provide additional support for further evaluation of lenabasum in CF in a larger, longer study. Limitations of this study were that there were only a small numbers of people who participated in the trial, and the study was short in duration. It is important to note that this was the first study evaluating lenabasum in people with CF and that a larger and longer study is required to confirm these initial safety and efficacy findings.



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## **What's next?**

We recently concluded another Phase 2 study which was specifically designed to look at the safety and efficacy of lenabasum in CF. This other study was much larger in size and longer in duration with 426 patients treated for six months. We look forward to sharing results from this study soon.

## **Original manuscript citation in PubMed**

<https://pubmed.ncbi.nlm.nih.gov/33011099/>

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