

Cystic Fibrosis Research News

Title:

Clinical effect of lumacaftor/ivacaftor in F508del homozygous CF patients with FEV₁ ≥90% predicted at baseline

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What was your research question?

Are there any clinical effects that could indicate if people with CF with well-preserved lung function (FEV₁ at least 90% predicted) benefit from lumacaftor/ivacaftor (Orkambi)?

Why is this important?

Lung function (measured with FEV₁) is, the most widely used method of tracking people's responses to lumacaftor/ivacaftor and is the most common outcome measure to help make decisions about treatment and reimbursement. However, changes in lung function may not be the best way to assess peoples' response to lumacaftor/ivacaftor if they already have well-preserved lung function. It is currently unclear whether this group of people could benefit in other ways from lumacaftor/ivacaftor treatment, e.g. improvements in nutritional state, fewer respiratory infections (needing a course antibiotics) or better quality of life.

What did you do?

We collected clinical information from people with CF who had well-preserved lung function and who started treatment with lumacaftor/ivacaftor. Lung function, a quality of life questionnaire, sweat test, height and weight were measured prior to starting treatment and after 6 months. Height, weight and lung function were also measured after 3, 9 and 12 months of treatment. All respiratory infections needing antibiotics were recorded. We compared the measurements at the start and the end of the study to evaluate the changes that occurred with treatment.

What did you find?



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Average lung function did not change during the year of treatment with lumacaftor/ivacaftor. However, nutritional state (measured in body mass index) improved, and fewer courses of antibiotic treatment were needed (almost half compared to the year before). Furthermore, the total scores on quality of life questionnaires improved. Sweat test improved even more than we expected based on previous studies about lumacaftor/ivacaftor.

What does this mean and reasons for caution?

People with CF with well-preserved lung function do benefit from lumacaftor/ivacaftor treatment in ways other than improved lung function.

It is not clear whether the improvement in quality of life score reflects an important change to patients' lives. This is because we used the entire questionnaire score in our study rather than limiting our analysis to the part about respiratory symptoms, as other studies have done. Also, because it is a costly treatment it will, at least in the short term, increase healthcare expenses. At this time, we could not investigate if the treatment would be cost-effective in the long term.

What's next?

We hope this study encourages caregivers and insurance companies to not only evaluate lung function response after starting treatment, but also nutritional state and respiratory infections, as these can be equally important. As the large response in sweat test surprised us, we have started additional research to find out why this occurred.

Original manuscript citation in PubMed

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