European Cystic Fibrosis Society

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Cystic Fibrosis Research News

Title:

VOCAL: An observational study of ivacaftor for people with cystic fibrosis and selected non–*G551D-CFTR* gating mutations

Lay Title:

Long-term health effects of real-world use of ivacaftor by people with cystic fibrosis who have non-G551D-CFTR gating mutations

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What was your research question?

We wanted to understand the long-term health effects for people with CF who have certain mutations and who take the CFTR modulator medication, ivacaftor, for long periods of time in the "real world" rather than as part of a research trial.

Why is this important?

Clinical research studies of modulator medications like ivacaftor suggest that they are generally safe and effective in treating the underlying cause of CF in patients with certain genetic mutations. However, modulator medications are not a cure for CF and need to be taken consistently in order to continue to treat the disease. We therefore need to learn as much as we can about the health effects of modulator medications like ivacaftor for people with CF who take them for long periods of time.

What did you do?

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The study followed health outcomes in people with CF and specific mutations for up to 48 months after starting ivacaftor. During the year before starting ivacaftor and up to 48 months afterward, we collected information about patients' lung function, weight, how often they were sick with pulmonary exacerbations, how many times they needed antibiotics, how often they were seen in clinic or admitted to the hospital, and what bacteria were found to grow in samples from their airways. We then compared how the patients did after starting ivacaftor with how they were doing before starting it.

What did you find?

We found patients' lung function improved and measures of bodyweight increased after starting ivacaftor. Likewise, patients also had fewer pulmonary exacerbations, needed fewer antibiotic courses, and grew bacteria in fewer samples from their airways while taking ivacaftor than they did before starting it. Patients also had fewer doctors' visits and were admitted to the hospital less often and for fewer days out of the year than before starting ivacaftor. These benefits of treatment continued for the duration of the study. Adverse events reported during this study were generally consistent with common effects of CF and the known safety profile of ivacaftor.

What does this mean and reasons for caution?

This study suggests that ivacaftor provides continued long-term benefits during real-world use for up to 48 months in people with CF and the specific mutations. Because this was an observational study, it was not possible to compare the findings in this group of patients taking ivacaftor with those of a similar group of patients not taking ivacaftor.

What's next?

This study shows the potential for modulator therapies to have long-lasting benefits for people with CF. To decrease the burden of CF on those affected, studies are looking at the effects of various modulator therapies in younger ages, over longer periods of time, and in patients with different genetic mutations.

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