Title:
Disease Progression in Patients with Cystic Fibrosis Treated with Ivacaftor: Data From National US and UK Registries

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What was your research question?
What are the effects of long-term treatment with a drug called ivacaftor on the progression of cystic fibrosis (CF) disease (how quickly the disease gets worse)?

Why is this important?
Ivacaftor was the first of a new class of drugs for treating people with CF. Because it is relatively new, there is not much information on the long-term impact of ivacaftor treatment when used in the real world. This study was a long-term follow-up of a large group of people treated with ivacaftor. There were more people included than in any previous study and by studying these peoples’ CF disease for up to 5 years, we have important new information about the impact of ivacaftor on the progression of CF disease over time.
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What did you do?

We obtained data from the existing CF patient registries in the United States and United Kingdom on people who were treated with ivacaftor from the first year the drug was available (2012 in the United States and 2013 in the United Kingdom) through to 2016. To evaluate the impact of ivacaftor, we compared these data to data from individuals of similar age, sex, and disease severity who did not receive ivacaftor treatment.

What did you find?

People treated with ivacaftor had better results than untreated people with CF. They maintained better lung function, had better nutritional results, had fewer pulmonary exacerbations and hospitalizations, and had favourable trends in the occurrence of CF-related diabetes and bacterial infections (with P. aeruginosa) throughout the 4- to 5-year period. Furthermore, while people were being treated with ivacaftor, they had fewer pulmonary exacerbations and hospitalizations than they did before they started ivacaftor treatment. We analyzed data from the United States and United Kingdom registries separately and saw similar trends in both sets of data.

What does this mean and reasons for caution?

Overall, our findings suggest that ivacaftor treatment slows the clinical course of disease progression in people with CF, meaning the disease does not get worse as quickly when people take ivacaftor. These results should be viewed with caution because the study design had certain in-built limitations, including unavoidable differences between those treated with ivacaftor and those who were untreated (eg, the groups had different types of mutations). Importantly, however, our results were consistent with results from previous studies of ivacaftor treatment that used different methods and evaluated different groups of patients.

What’s next?

This study highlights the need for additional high-quality studies to continue to gain a better understanding of the real-world impact of ivacaftor treatment. More studies are needed to look at specific aspects of CF disease progression related to the lung and outside the lung.

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