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Cystic Fibrosis Research News

Title:

Real-world safety and effectiveness of elexacaftor/tezacaftor/ivacaftor in people with cystic fibrosis: interim results of a long-term registry-based study

Lay Title:

Two-year follow-up results from an ongoing study on the safety and effectiveness of elexacaftor/tezacaftor/ivacaftor when used in real-world settings by people with cystic fibrosis

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What was your research question?

We wanted to know how the health of people with CF was affected when they were taking the CFTR modulator medication, elexacaftor/tezacaftor/ivacaftor, in the "real world" rather than as part of a clinical trial.

Why is this important?

Clinical trials of elexacaftor/tezacaftor/ivacaftor showed it is generally safe and effective in treating the underlying cause of CF in people with certain genetic mutations. However, it is also important to collect and study information on changes in health outcomes from people with CF who use elexacaftor/tezavaftor/ivacaftor outside the clinical trial setting, under real world conditions.

What did you do?

We followed people with CF for up to 2 years after starting elexacaftor/tezacaftor/ivacaftor and regularly measured certain health outcomes. For the 5-year period before starting elexacaftor/tezacaftor/ivacaftor and for up to 2 years afterward, we gathered information on lung function, weight, how often patients had pulmonary exacerbations, how often patients were hospitalized, other diagnosed health conditions, and bacteria found in samples from their airways. We then compared how people did after starting

elexacaftor/tezacaftor/ivacaftor with how they were doing before starting. Additionally, we collected information on organ transplants and deaths after starting elexacaftor/tezacaftor/ivacaftor.

What did you find?

We found that lung function improved and measures of body weight increased after people started taking elexacaftor/tezacaftor/ivacaftor. Additionally, people had fewer pulmonary exacerbations and grew less bacteria from airway samples while taking elexacaftor/tezacaftor/ivacaftor than before starting it. People were also admitted to the hospital less often than before starting elexacaftor/tezacaftor/ivacaftor. These benefits continued for the duration of the study. The rates of death and lung transplantation were lower after starting elexacaftor/tezacaftor/ivacaftor when compared with a similar group of people with CF who did not take elexacaftor/tezacaftor/ivacaftor during the time-period just before elexacaftor/tezacaftor/ivacaftor became commercially available.

What does this mean and reasons for caution?

These findings suggest that when people with CF and specific mutations take elexacaftor/tezacaftor/ivacaftor there are similar benefits in the real world as were seen in clinical trials up to the 2-year follow up point. As this was an observational study, we could not compare our findings in this group of people taking elexacaftor/tezacaftor/ivacaftor with those from a similar group of people not taking elexacaftor/tezacaftor/ivacaftor at the same time.

What's next?

The study will continue for another 3 years, allowing us to collect more information on the long-term impact of using elexacaftor/tezacaftor/ivacaftor in the real world on health outcomes in people with CF.

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