

Cystic Fibrosis Research News

Title:

WILLINGNESS OF PEOPLE WITH CYSTIC FIBROSIS RECEIVING ELEXACAFTOR/TEZACAFTOR/IVACAFTOR (ETI) TO PARTICIPATE IN RANDOMIZED MODULATOR AND INHALED ANTIMICROBIAL CLINICAL TRIALS

Lay Title:

Willingness of people taking elxacaftor/tezacaftor/ivacaftor to be in new CFTR modulator studies

Authors:

D.R. VanDevanter,^{1*} E.T. Zemanick,² M.W. Konstan,^{1,3} C.L. Ren,⁴ K. Odem-Davis,⁵ I. Emerman,⁵ J. Young,⁵ N. Mayer-Hamblett,^{5,6}

Affiliations:

¹Case Western Reserve University School of Medicine, Cleveland, OH United States,

²University of Colorado, Anschutz Medical Campus, Aurora CO United States

³Rainbow Babies and Children's Hospital, Cleveland, OH United States

⁴Children's Hospital of Philadelphia, Philadelphia, PA United States

⁵Seattle Children's Hospital, Seattle, WA United States

⁶University of Washington, Seattle, WA United States

What was your research question?

Would people with CF currently taking the CFTR modulator elxacaftor/tezacaftor/ivacaftor be willing to enroll in clinical studies of a new modulator? How would study design (for instance, if some participants were asked to take stop taking modulators) or study length (2 weeks versus longer times) affect their interest?

Why is this important?

Some people who have the right kind of CF mutations to be able to take elxacaftor/tezacaftor/ivacaftor can't take it, either because it makes them sick, they can't afford it, or because it is not available in their area. CF researchers want to find new modulators that work as well as elxacaftor/tezacaftor/ivacaftor to make it easier for these people to benefit from modulators. Showing that a new modulator works well and is safe would take more people than just those who can't take elxacaftor/tezacaftor/ivacaftor today, it would also need some people currently taking elxacaftor/tezacaftor/ivacaftor to enroll in studies.



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What did you find?

Most people taking elexacaftor/tezacaftor/ivacaftor said they would enroll in new CFTR modulator studies, but the study design had a big effect on their answers. People were less interested in studies where they might be asked to take a sugar pill (placebo) instead of the new modulator and more interested in studies where they would be asked to take either the new modulator or elexacaftor/tezacaftor/ivacaftor. People were much more interested in shorter studies (a month or less) than longer studies (up to 6 months). Most people taking both elexacaftor/tezacaftor/ivacaftor and an inhaled antibiotic would join studies of a new inhaled antibiotic.

What does this mean and reasons for caution?

Our results mean researchers can probably run studies big enough to show that a new modulator works and is effective because most people taking elexacaftor/tezacaftor/ivacaftor say they will participate, provided that those studies are not too long and don't ask people to go off of modulator treatment. We need to be cautious in using these results to design studies because people taking elexacaftor/tezacaftor/ivacaftor that told us they would enroll in a future study might decide not to enroll in one once the risks involved in a study design are fully explained to them.

What's next?

CF researchers are testing new CFTR modulators. Our results will help researchers design studies with enough people to show that new modulators work and are safe. They will also help people responsible for approving drugs (regulators) to understand which study designs will be able to enroll people taking elexacaftor/tezacaftor/ivacaftor.

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