Cystic Fibrosis Research News

Title:

A Phase 3, Double-Blind, Parallel-Group Study to Evaluate the Efficacy and Safety of Tezacaftor in Combination With Ivacaftor in Participants 6 Through 11 Years of Age With Cystic Fibrosis Homozygous for *F508del* or Heterozygous for the *F508del-CFTR* Mutation and a Residual Function Mutation

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What was your research question?

Is a drug called tezacaftor/ivacaftor safe and does it work well to treat people aged 6 through 11 years old with cystic fibrosis (CF) who carry either 2 copies of the *F508del-CFTR* mutation or one *F508del-CFTR* mutation and one type of *CFTR* mutation called a residual function (RF) mutation?

Why is this important?

Tezacaftor/ivacaftor has been tested in people with 2 copies of the *F508del-CFTR* mutation or one *F508del-CFTR* mutation and one RF mutation. Tezacaftor/ivacaftor has been shown to work well and be safe in people 12 years old or older with these mutations. It has also been shown to be safe in people aged 6 through 11 years old with these mutations, but no studies have looked at how well it works in this age group with these mutations.

What did you do?

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In this Phase 3, randomized, double-blind study, people aged 6 through 11 years old with CF who had 2 copies of the *F508del-CFTR* mutation or one *F508del-CFTR* mutation and one RF mutation took tezacaftor/ivacaftor for up to 8 weeks. We looked at how well this drug worked to treat CF, how safe it was, and whether people taking the drug could tolerate it (didn't have to stop taking it because of side effects).

What did you find?

We found that tezacaftor/ivacaftor worked well at treating people aged 6 through 11 years old with CF who had these mutations. Tezacaftor/ivacaftor was generally safe for people in this study and did not have side effects that were hard to manage.

What does this mean and reasons for caution?

The results of this study show that tezacaftor/ivacaftor works well to treat people aged 6 through 11 years old with CF with 2 copies of the *F508del-CFTR* mutation or one *F508del-CFTR* mutation and one RF mutation. These results also further show that tezacaftor/ivacaftor is safe in people with CF in this age group and with these types of mutations and does not cause side effects that are hard to manage.

What's next?

Elexacaftor combined with tezacaftor/ivacaftor is approved in the USA for people 12 years old and older with CF with one or more *F508del-CFTR* mutation. Studies are looking at whether elexacaftor/tezacaftor/ivacaftor works well and is safe in people aged 6 through 11 years old with 2 copies of *F508del-CFTR* or *F508del-CFTR* and a RF mutation.

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