

Cystic Fibrosis Research News

Title:

A Phase 3, Open-Label, 96-Week Trial to Study the Safety, Tolerability, and Efficacy of Tezacaftor/Ivacaftor in Children ≥ 6 Years of Age Homozygous for *F508del* or Heterozygous for *F508del* and a Residual Function *CFTR* Variant

Lay Title:

Tezacaftor/Ivacaftor in Children ≥ 6 Years of Age With *F508del* and Residual Function Changes in *CFTR*

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What was your research question?

Is the drug tezacaftor/ivacaftor safe, and does it work well in children with cystic fibrosis (CF) who have either (a) 2 copies of a change in the *CFTR* gene that causes CF, called *F508del-CFTR*, or (b) 1 copy of *F508del-CFTR* plus another change called a residual function variant?

Why is this important?

Earlier studies showed that tezacaftor/ivacaftor is generally safe and works well for up to 24 weeks in children 6 through 11 years of age who have either 2 copies of *F508del-CFTR* or 1 copy of *F508del-CFTR* plus a residual function variant. It has also been shown to be safe and work well over a longer period (up to 96 weeks) in older people with CF. However, no studies had looked at whether tezacaftor/ivacaftor is safe and works well over a longer period in children as young as 6 years of age.

What did you do?

Children 6 years of age or older with CF who had either 2 copies of *F508del-CFTR* or 1 copy of *F508del-CFTR* plus a residual function variant and who previously took tezacaftor/ivacaftor in an earlier 8-week or 24-week study took tezacaftor/ivacaftor for up to 96 weeks. We looked at (a) how safe tezacaftor/ivacaftor was, (b) if children could tolerate the drug (ie, they didn't have to stop taking it because of side effects), and (c) how well it worked to treat CF.

What did you find?

Tezacaftor/ivacaftor was generally safe for children 6 years and older in this study and did not have side effects that were hard to manage. Tezacaftor/ivacaftor worked well to treat CF: the same improvements observed in the earlier studies were observed in this study and they were maintained over the additional 96 weeks.

What does this mean and reasons for caution?

The results of this study show that tezacaftor/ivacaftor is safe and works well to treat children with CF aged 6 years and older who have either 2 copies of *F508del-CFTR* or 1 copy of *F508del-CFTR* plus a residual function variant. These findings support the long-term use of tezacaftor/ivacaftor in this population.



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What's next?

Tezacaftor/ivacaftor is approved by the US Food and Drug Administration and European Commission to treat CF in people 6 years and older who have certain *CFTR* gene changes. Studies are looking at whether tezacaftor/ivacaftor is safe and works well with long-term use in the real-world setting (outside of controlled studies).

Original manuscript citation in PubMed

<https://pubmed.ncbi.nlm.nih.gov/35190292/>

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