primary studies - published RCT

Tezacaftor-Ivacaftor in Residual-Function Heterozygotes with Cystic Fibrosis.

Code: PM29099333 **Year:** 2017 **Date:** 2017 **Author:** Rowe SM

Study design (if review, criteria of inclusion for studies)

Randomized, double-blind, placebo-controlled, phase 3, crossover trial

Participants

248 patients 12 years of age or older who had cystic fibrosis and were heterozygous for the Phe508del mutation and a CFTR mutation associated with residual CFTR function.

Interventions

Patients were randomly assigned to one of six sequences, each involving two 8-week intervention periods separated by an 8-week washout period. They received tezacaftor-ivacaftor, ivacaftor monotherapy, or placebo.

Outcome measures

The primary end point was the absolute change in the percentage of predicted forced expiratory volume in 1 second (FEV1) from the baseline value to the average of the week 4 and week 8 measurements in each intervention period.

Main results

The number of analyzed intervention periods was 162 for tezacaftor-ivacaftor, 157 for ivacaftor alone, and 162 for placebo. The least-squares mean difference versus placebo with respect to the absolute change in the percentage of predicted FEV1 was 6.8 percentage points for tezacaftor-ivacaftor and 4.7 percentage points for ivacaftor alone (P

Authors' conclusions

CFTR modulator therapy with tezacaftor-ivacaftor or ivacaftor alone was efficacious in patients with cystic fibrosis who were heterozygous for the Phe508del deletion and a CFTR residual-function mutation. (Funded by Vertex Pharmaceuticals and others; EXPAND ClinicalTrials.gov number, NCT02392234 .).

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See also

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Keywords

Adult; Aged; CFTR Modulators; Genetic Predisposition to Disease; pharmacological_intervention; placebo; VX-770; VX-661; ivacaftor; Aminophenols; tezacaftor; Symdeko; Symkevi;