

primary studies - published RCT

Tezacaftor-Ivacaftor in Patients with Cystic Fibrosis Homozygous for Phe508del.

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

Phase 3, randomized, double-blind, multicenter, placebo-controlled, parallel-group trial

Participants

Patients 12 years of age or older who had cystic fibrosis and were homozygous for the CFTR Phe508del mutation.

Interventions

Patients were randomly assigned in a 1:1 ratio to receive either 100 mg of tezacaftor once daily and 150 mg of ivacaftor twice daily or matched placebo for 24 weeks.

Outcome measures

The primary end point was the absolute change in the percentage of the predicted forced expiratory volume in 1 second (FEV1) through week 24 (calculated in percentage points); relative change in the percentage of the predicted FEV1 through week 24 (calculated as a percentage) was a key secondary end point.

Main results

Of the 510 patients who underwent randomization, 509 received tezacaftor-ivacaftor or placebo, and 475 completed 24 weeks of the trial regimen. The mean FEV1 at baseline was 60.0% of the predicted value. The effects on the absolute and relative changes in the percentage of the predicted FEV1 in favor of tezacaftor-ivacaftor over placebo were 4.0 percentage points and 6.8%, respectively (P

Authors' conclusions

The combination of tezacaftor and ivacaftor was efficacious and safe in patients 12 years of age or older who had cystic fibrosis and were homozygous for the CFTR Phe508del mutation.

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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;