primary studies - published RCT

Elexacaftor-Tezacaftor-Ivacaftor for Cystic Fibrosis with a Single Phe508del Allele.

Code: PM31697873 **Year:** 2019 **Date:** 2019 **Author:** Middleton PG

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

Phase 3, randomized, double-blind, placebo-controlled trial

Participants

Patients 12 years of age or older with cystic fibrosis with Phe508del-minimal function genotypes.

Interventions

Patients were randomly assigned to receive elexacaftor-tezacaftor-ivacaftor or placebo for 24 weeks.

Outcome measures

The primary end point was absolute change from baseline in percentage of predicted forced expiratory volume in 1 second (FEV1) at week 4.

Main results

A total of 403 patients underwent randomization and received at least one dose of active treatment or placebo. Elexacaftor-tezacaftor-ivacaftor, relative to placebo, resulted in a percentage of predicted FEV1 that was 13.8 points higher at 4 weeks and 14.3 points higher through 24 weeks, a rate of pulmonary exacerbations that was 63% lower, a respiratory domain score on the Cystic Fibrosis Questionnaire-Revised (range, 0 to 100, with higher scores indicating a higher patient-reported quality of life with regard to respiratory symptoms; minimum clinically important difference, 4 points) that was 20.2 points higher, and a sweat chloride concentration that was 41.8 mmol per liter lower (P

Authors' conclusions

Elexacaftor-tezacaftor-ivacaftor was efficacious in patients with cystic fibrosis with Phe508del-minimal function genotypes, in whom previous CFTR modulator regimens were ineffective.

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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; VX-445; elexacaftor; Trikafta; kaftrio;