primary studies - published RCT

Tezacaftor/ivacaftor in people with cystic fibrosis heterozygous for minimal function CFTR mutations.

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

This randomized, double-blind, placebo-controlled Phase 3 study

Participants

AB - BACKGROUND: Tezacaftor/ivacaftor is a CFTR modulator approved to treat people with cystic fibrosis (pwCF) who are homozygous (F/F) or heterozygous for the F508del-CFTR mutation and a residual function mutation (F/RF).

Interventions

Participants were randomized 1:1 to receive tezacaftor/ivacaftor or placebo for 12 weeks.

Outcome measures

The primary endpoint was the absolute change from baseline in percent predicted forced expiratory volume in 1 second (ppFEV(1)) between the tezacaftor/ivacaftor and placebo groups through week 12. Key secondary endpoints included absolute change from baseline in CF Questionnaire-Revised respiratory domain scores and the number of pulmonary exacerbations through week 12 and the absolute change from baseline in body mass index at week 12.

Main results

At the time of the IA, 83 participants were randomized to tezacaftor/ivacaftor and 85 to placebo; 165 participants completed treatment. The study failed to demonstrate that tezacaftor/ivacaftor significantly improved ppFEV(1) or any of the key secondary endpoints and was terminated for futility. The safety profile and PK parameters of tezacaftor/ivacaftor were similar to those reported in prior studies in participants â‰¥12 years of age with CF.

Authors' conclusions

Tezacaftor/ivacaftor did not show a clinically meaningful benefit in participants with F/MF genotypes but was generally safe and well tolerated, consistent with the safety profile reported in other Phase 3 studies

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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;