Tezacaftor/Ivacaftor in Subjects with Cystic Fibrosis and F508del/F508del-CFTR or F508del/G551D-CFTR.

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

Randomized, placebo-controlled, double-blind, multicenter, phase 2 study

Participants

Subjects homozygous for F508del. Subjects compound heterozygous for F508del and G551D

Interventions

Subjects homozygous for F508del received tezacaftor (10 mg to 150 mg) qday alone or in combination with ivacaftor 150 mg q12h in a dose escalation phase, as well as in a dosage regimen testing phase. Subjects compound heterozygous for F508del and G551D taking physician prescribed ivacaftor received tezacaftor 100 mg qday.

Outcome measures

Primary endpoints were safety through day 56 and change in sweat chloride from baseline through day 28. Secondary endpoints included change in percent predicted FEV1 (ppFEV1) from baseline through day 28 and pharmacokinetics. Incidence of adverse events

Main results

The incidence of adverse events was similar across treatment arms. Tezacaftor 100 mg qday/ivacaftor 150 mg q12h resulted in a 6.04 mmol/L decrease in sweat chloride and 3.75 percentage point increase in ppFEV1 in subjects homozygous for F508del and a 7.02 mmol/L decrease in sweat chloride and 4.60 percentage point increase in ppFEV1 in subjects compound heterozygous for F508del and G551D from baseline through day 28.

Authors’ conclusions

These results support continued clinical development of tezacaftor 100 mg qday in combination with ivacaftor 150 mg q12h in subjects with CF.

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See also


Keywords

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