primary studies - published RCT

Efficacy and safety of ivacaftor in patients with cystic fibrosis who have an Arg117His-CFTR mutation: a double-blind, randomised controlled trial.

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

placebo-controlled, double-blind, randomised clinical trial. Open-label extension enrolled 65 of the patients after washout; interim analysis after 12 weeks.

Participants

69 patients with cystic fibrosis aged 6 years and older with Arg117His-CFTR and percentage of predicted forced expiratory volume in 1 s (% predicted FEV1) of at least 40.

Interventions

Patients were randomly assigned (1:1) to receive placebo or ivacaftor 150 mg every 12 h for 24 weeks. Randomisation was stratified by age (6-11, 12-17, and >/=18 years) and % predicted FEV1 (<70, >/=70 to </=90, and >90).

Outcome measures

The primary outcome was the absolute change from baseline in % predicted FEV1 through week 24. Secondary outcomes included safety and changes in sweat chloride concentrations and Cystic Fibrosis Questionnaire-Revised (CFQ-R) respiratory domain scores.

Main results

After 24 weeks, the treatment difference in mean absolute change in % predicted FEV1 between ivacaftor (n=34) and placebo (n=35) was 2.1 percentage points (95% CI -1.13 to 5.35; p=0.20). Ivacaftor treatment resulted in significant treatment differences in sweat chloride (-24.0 mmol/L, 95% CI -28.01 to -19.93; p

Authors' conclusions

Although this study did not show a significant improvement in % predicted FEV1, ivacaftor did significantly improve sweat chloride and CFQ-R respiratory domain scores and lung function in adult patients with Arg117His-CFTR, indicating that ivacaftor might benefit patients with Arg117His-CFTR who have established disease. FUNDING: Vertex Pharmaceuticals Incorporated.

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See also

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Keywords

CFTR Modulators; pharmacological_intervention; VX-770; ivacaftor; Genetic Predisposition to Disease; Aminophenols;