primary studies - published RCT

A CFTR potentiator in patients with cystic fibrosis and the G551D mutation.

Code: PM22047557 **Year:** 2011 **Date:** 2011 **Author:** Ramsey BW

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

randomized, double-blind, placebo-controlled trial

Participants

subjects 12 years of age or older with cystic fibrosis and at least one G551D-CFTR mutation.

Interventions

Subjects were randomly assigned to receive 150 mg of ivacaftor every 12 hours (84 subjects, of whom 83 received at least one dose) or placebo (83, of whom 78 received at least one dose) for 48 weeks.

Outcome measures

The primary end point was the estimated mean change from baseline through week 24 in the percent of predicted forced expiratory volume in 1 second (FEV(1))

Main results

The change from baseline through week 24 in the percent of predicted FEV(1) was greater by 10.6 percentage points in the ivacaftor group than in the placebo group (P

Authors' conclusions

Ivacaftor was associated with improvements in lung function at 2 weeks that were sustained through 48 weeks. Substantial improvements were also observed in the risk of pulmonary exacerbations, patient-reported respiratory symptoms, weight, and concentration of sweat chloride.

http://dx.doi.org/10.1056/NEJMoa1105185

See also

N Engl J Med. 2011 Nov 3;365(18):1663-72.

Keywords

Adult; Aminophenols; Anti-Bacterial Agents; CFTR Modulators; G551D-CFTR; Genetic Predisposition to Disease; pharmacological_intervention; Quinolones; VX-770; ivacaftor;