primary studies - published RCT

Efficacy and Safety of Ivacaftor in Patients Aged 6 to 11 Years with Cystic Fibrosis with a G551D Mutation.

Code: PM23590265 **Year:** 2013 **Date:** 2013 **Author:** Davies JC

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

Randomized, double-blind, placebo-controlled trial

Participants

Patients with CF aged 6-11 years with a G551D-CFTR mutation on at least one allele.

Interventions

Patients were randomly assigned to receive ivacaftor administered orally at 150 mg (n=26) or placebo (n=26) every 12 hours for 48 weeks in addition to existing prescribed cystic fibrosis therapies.

Outcome measures

Forced expiratory volume in 1 second (FEV1); concentration of sweat chloride; incidence of adverse events

Main results

Despite near normal mean baseline values in forced expiratory volume in 1 second (FEV1), patients receiving ivacaftor had a significant increase in percent predicted FEV1 from baseline through Week 24 versus placebo group (treatment effect: 12.5 percentage points; P

Authors' conclusions

In patients who are younger and healthier than those in previously studied populations, ivacaftor demonstrated a meaningful improvement in pulmonary function, weight, and CFTR activity compared with placebo.

http://dx.doi.org/10.1164/rccm.201301-0153OC

See also

Am J Respir Crit Care Med. 2013 Apr 3.

Keywords

Child; Aminophenols; CFTR Modulators; Genetic Predisposition to Disease; pharmacological_intervention; VX-770; ivacaftor; G551D-CFTR;