primary studies - published RCT

Efficacy and safety of ivacaftor in patients with cystic fibrosis and a non-G551D gating mutation

Code: PM25266159 **Year:** 2014 **Date:** 2014 **Author:** De Boeck K

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

Phase 3 crossover RCT

Participants

39 people aged six and older with at least one non-G551D gating mutation. receivedThe primary efficacy outcome was absolute change in FEV1 through 8 and 24weeks of ivacaftor treatment; secondary outcomes were changes in BMI, sweat chloride, and CFQ-R and safety through 8 and 24weeks of treatment.

Interventions

ivacaftor 150mg q12h or placebo for 8weeks in this 2-part, double-blind crossover study (Part 1) with a 16-week open-label extension (Part 2).

Outcome measures

The primary efficacy outcome was absolute change in FEV1 through 8 and 24weeks of ivacaftor treatment; secondary outcomes were changes in BMI, sweat chloride, and CFQ-R and safety through 8 and 24weeks of treatment.

Main results

Eight weeks of ivacaftor resulted in significant improvements in percent predicted FEV1, BMI, sweat chloride, and CFQ-R scores that were maintained through 24weeks. Ivacaftor was generally well tolerated.

Authors' conclusions

Ivacaftor was efficacious in a group of patients with CF who had selected non-G551D gating mutations.

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See also

J Cyst Fibros. 2014 Dec;13(6):674-80.

Keywords

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