primary studies - published RCT

Lumacaftor/Ivacaftor Treatment of Patients with Cystic Fibrosis Heterozygous for F508del-CFTR.

Code: PM27898234 **Year:** 2017 **Date:** 2017 **Author:** Rowe SM

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

RCT

Participants

Patients heterozygous for F508del-CFTR, aged 18 years or older with a confirmed CF diagnosis and percent predicted FEV1 (ppFEV1) of 40 to 90

Interventions

Patients were randomized to lumacaftor/ivacaftor (400 mg/250 mg every 12 h) or placebo daily for 56 days.

Outcome measures

Primary outcomes were change in ppFEV1 at Day 56 and safety.

Main results

Of 126 patients, 119 (94.4%) completed the study. Lumacaftor/ivacaftor was well tolerated, although chest tightness and dyspnea occurred more frequently with active treatment than with placebo (27.4% vs. 14.3% and 14.5% vs. 6.3%, respectively). Mean (SD) ppFEV1 values at baseline were 62.9 (14.3) in the active treatment group and 60.1 (14.0) in the placebo group. Absolute changes in ppFEV1 (least squares mean [SE]) at Day 56 were -0.6 (0.8) percentage points in the active treatment group and -1.2 (0.8) percentage points in the placebo group (P = 0.60). CF respiratory symptom scores in the active treatment group improved by a mean of 5.7 points versus a decrease of -0.8 in the placebo group (P

Authors' conclusions

Sweat chloride and respiratory symptom scores improved with lumacaftor/ivacaftor, though no meaningful benefit was seen in ppFEV1 or body mass index in patients heterozygous for F508del-CFTR.

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See also

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Keywords

Child; Adult; Adolescent; Aminophenols; CFTR Modulators; Genetic Predisposition to Disease; Orkambi; pharmacological_intervention; VX-770; ivacaftor; lumacaftor; VX-809;