

primary studies - published RCT

## Lumacaftor-Ivacaftor in Patients with Cystic Fibrosis Homozygous for Phe508del CFTR.

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

Two phase 3, randomized, double-blind, placebo-controlled studies ; TRAFFIC and TRANSPORT

### Participants

Patients 12 years of age or older who had cystic fibrosis and were homozygous for the Phe508del CFTR mutation.

### Interventions

Patients were randomly assigned to receive either lumacaftor (600 mg once daily or 400 mg every 12 hours) in combination with ivacaftor (250 mg every 12 hours) or matched placebo for 24 weeks.

### Outcome measures

The primary end point was the absolute change from baseline in the percentage of predicted forced expiratory volume in 1 second (FEV1) at week 24. Secondary outcomes: the rate of pulmonary exacerbations, the rate of events leading to hospitalization or the use of intravenous antibiotics, the incidence of adverse events .

### Main results

A total of 1108 patients underwent randomization and received study drug. The mean baseline FEV1 was 61% of the predicted value. In both studies, there were significant improvements in the primary end point in both lumacaftor-ivacaftor dose groups; the difference between active treatment and placebo with respect to the mean absolute improvement in the percentage of predicted FEV1 ranged from 2.6 to 4.0 percentage points (P

### Authors' conclusions

These data show that lumacaftor in combination with ivacaftor provided a benefit for patients with cystic fibrosis homozygous for the Phe508del CFTR mutation. (Funded by Vertex Pharmaceuticals and others)

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### See also

N Engl J Med. 2015 May 17.

### Keywords

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