primary studies - published RCT

Assessment of clinical response to ivacaftor with lung clearance index in cystic fibrosis patients with a G551D-CFTR mutation and preserved spirometry: a randomised controlled trial

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

Phase 2, multicentre, placebo-controlled, double-blind 2×2 crossover study. Randomisation (ratio 1:1) was done with block sizes of 4, and all site personnel including the investigator, the study monitor, and the Vertex study team were masked to treatment assignment.

Participants

21 Patients with cystic fibrosis, at least one G551D-CFTR allele, and an FEV1 >90% predicted. Patients also had to have an LCI higher than 7*4 at screening, age of 6 years or older, and a weight higher than or equal to 15 kg.

Interventions

Patients were randomly allocated to receive one of two treatment sequences (placebo first followed by ivacaftor 150 mg twice daily [sequence 1] or ivacaftor 150 mg twice daily first followed by placebo [sequence 2]) of 28 days' treatment in each period, with a 28-day washout between the two treatment periods.

Outcome measures

The primary outcome measure was change from baseline in LCI.

Main results

21 patients were enrolled, of which 11 were assigned to the sequence 1 group, and 10 to the sequence 2 group. 20 of these patients received treatment and 17 completed the trial (eight in sequence 1 group and 9 in sequence 2 group). Treatment with ivacaftor led to significant improvements compared with placebo in LCI (difference between groups in the average of mean changes from baseline at days 15 and 29 was â^'2*16 [95% CI â^'2*88 to â^'1*44]; p

Authors' conclusions

In patients with cystic fibrosis aged 6 years or older who have at least one G551D-CFTR allele, ivacaftor led to improvements in LCI. LCI might be a more sensitive alternative to FEV1 in detecting response to intervention in these patients with mild lung disease.

http://www.thelancet.com/journals/lanres/article/PIIS2213-2600%2813%2970182-6/abstract

See also

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Keywords

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