primary studies - published RCT

Ivacaftor in cystic fibrosis with residual function: Lung function results from an N-of-1 study.

Code: PM31784217 **Year:** 2020 **Date:** 2020 **Author:** Nick JA

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

Randomized double-blind crossover trial.

Participants

Patients aged â‰¥12 years with CF with clinical or molecular evidence of residual CFTR function

Interventions

Patients were randomized to 1 of 4 treatment sequences for two 4-week, double-blind crossover cycles (each divided into 2 weeks of ivacaftor treatment and placebo) followed by 8 weeks of open-label ivacaftor treatment.

Outcome measures

The primary endpoint was absolute change from cycle baseline of percent predicted forced expiratory volume in 1 s (ppFEV(1)) after 2 weeks of treatment with ivacaftor relative to placebo.

Main results

Absolute change (SD) from study baseline in ppFEV(1) favored ivacaftor by 2.3 (1.0) percentage points (95% credible interval, 0.4-4.1) after 2 weeks of treatment. Absolute mean change (SD) from open-label baseline (defined as day 1 of the open-label ivacaftor treatment period) in ppFEV(1) after 8 weeks of treatment was 4.7 (4.2) percentage points (P

Authors' conclusions

Ivacaftor improved lung function during the double-blind and open-label treatment periods in patients with CF and CFTR mutations associated with residual CFTR function.

http://dx.doi.org/10.1016/j.jcf.2019.09.013

See also

J Cyst Fibros. 2020 Jan;19(1):91-98. doi: 10.1016/j.jcf.2019.09.013. Epub 2019 Nov 26.

Keywords

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