primary studies - published RCT

Improvement in exercise duration, lung function and well-being in G551D-Cystic Fibrosis patients: a double-blind, placebo-controlled, randomised, cross-over study with ivacaftor.

Code: PM28611235 **Year:** 2017 **Date:** 2017 **Author:** Keating DT

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

Single-centre, double-blind, placebo-controlled, 28-day crossover study of ivacaftor.

Participants

Twenty G551D-CF patients.

Interventions

Ivacaftor

Outcome measures

Exercise capacity . Variables measured included percentage change from baseline (%Delta) of VO2max (maximal oxygen consumption, primary outcome) during cardiopulmonary exercise testing (CPET), relevant other CPET physiological variables, lung function, BMI, sweat chloride, and disease specific health related quality of life (QOL) measures (CFQ-R and Alfred Wellness (AWEscore)). %DeltaVO2max was unchanged compared to placebo as was %Deltaminute ventilation.

Main results

%Deltaexercise time (mean 7.3, CI 0.5-14,1, p=0.0222) significantly increased as did %DeltaFEV1 (11.7%, range 5.3-18.1, p

Authors' conclusions

Investigation over a more prolonged period may delineate the potential interdependencies of the observed discordances over time.

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See also

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Keywords

Aminophenols; CFTR Modulators; Genetic Predisposition to Disease; pharmacological_intervention; VX-770; ivacaftor; G551D-CFTR;