
primary studies - published RCT

Body composition and weight changes after ivacaftor treatment in adults with cystic fibrosis carrying the G551 D cystic fibrosis transmembrane conductance regulator mutation: A double-blind, placebo-controlled, randomized, crossover study with open-label extension.

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

Single-center, double-blind, placebo-controlled, 28-d, crossover study, followed by an open-label extension (OLE) for 5 mo.

Participants

Twenty adults with CF carrying the G551D mutation (mean \pm standard deviation body mass index [BMI] 23.3 ± 4.3 kg/m²). Eleven patients underwent measurements 2 y later.

Interventions

Ivacaftor

Outcome measures

Weight, BMI, and body composition (including fat-free mass [FFM] and fat mass).

Main results

After 28 d of treatment with ivacaftor, weight increased by 1.1 ± 1.3 kg, BMI by 0.4 ± 0.5 kg/m², and FFM by 1.1 ± 1.2 kg (all P

Authors' conclusions

Small gains were seen in FFM in the first month of ivacaftor treatment. Weight, BMI, and fat-mass gains in the first 6 mo on ivacaftor plateaued by 2.5 y. The metabolic and clinical consequences of weight and fat-mass gains remain to be determined.

<http://dx.doi.org/10.1016/j.nut.2020.111124>

See also

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Keywords

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