primary studies - published RCT

Nutritional Status Improved in Cystic Fibrosis Patients with the G551D Mutation After Treatment with Ivacaftor.

Code: PM26250833 **Year:** 2016 **Date:** 2016 **Author:** Borowitz D

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

RCT

List of included studies

x

Participants

patients aged >6 years with CF and the G551D mutation.

Interventions

Patients were randomized 1:1 to ivacaftor 150 mg or placebo every 12 h for 48 weeks.

Outcome measures

Primary end point (lung function) was reported previously. Other outcomes included weight and height measurements and CF Questionnaire-Revised (CFQ-R).

Main results

Studies included 213 patients (aged </= 20 years, n = 105; aged > 20 years, n = 108). In patients </=20 years, adjusted mean change from baseline to week 48 in body weight was 4.9 versus 2.2 kg (ivacaftor vs. placebo, p = 0.0008). At week 48, change from baseline in mean weight-for-age z-score was 0.29 versus -0.06 (p < 0.0001); change in mean BMI-for-age z-score was 0.26 versus -0.13 (p < 0.0001). In patients >20 years, adjusted mean change from baseline to week 48 in body weight was 2.7 versus -0.2 kg (p = 0.0003). Mean BMI change at week 48 was 0.9 versus -0.1 kg/m(2) (p = 0.0003). There was no linear correlation evident between changes in body weight and improvements in lung function or sweat chloride. Significant CFQ-R improvements were seen in perception of eating, body image, and sense of ability to gain weight.

Authors' conclusions

Nutritional status improved following treatment with ivacaftor for 48 weeks.

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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;