

primary studies - published RCT

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

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### 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  $\leq 20$  years,  $n = 105$ ; aged  $> 20$  years,  $n = 108$ ). In patients  $\leq 20$  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<sup>2</sup> ( $p = 0.0003$ ). 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.

<http://dx.doi.org/10.1007/s10620-015-3834-2>

### See also

Dig Dis Sci. 2016 Jan;61(1):198-207. doi: 10.1007/s10620-015-3834-2. Epub 2015 Aug 7.

### Keywords

Aminophenols; CFTR Modulators; Genetic Predisposition to Disease; pharmacological\_intervention; VX-770; ivacaftor; G551D-CFTR;