*primary studies - published RCT*

# Effect of ivacaftor treatment in patients with cystic fibrosis and the G551D-CFTR mutation: Patient-reported outcomes in the STRIVE randomized, controlled trial.

**Code:** PM26135562 **Year:** 2015 **Date:** 2015 **Author:** Quittner A

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

Double-blind, placebo-controlled randomized trial,

### Participants

CF patients aged 12+ with the G551D-CFTR mutation

### Interventions

Ivacaftor (150 mg) for 48 weeks vs placebo

### Outcome measures

Treatment benefit analyses applied the cumulative distribution function and a categorical analysis of change scores ("improvement," "no change," or "decline"). Content-based interpretation examined treatment effect on specific item responses.

### Main results

Data from 152 patients with a baseline CFQ-R assessment were analyzed. The treatment effect analysis favored treatment with ivacaftor over placebo on the Body Image, Eating, Health Perceptions, Physical Functioning, Respiratory, Social Functioning, Treatment Burden, and Vitality scales. Findings were supported by the analysis of categorical change. On all CFQ-R scales, the percentage of patients who improved was greater for ivacaftor. In the content-based analysis, the treatment benefit was characterized by better scores across a broad range of domains.

### Authors' conclusions

Results illustrate broad benefits of ivacaftor treatment across many domains: respiratory symptoms, physical and social functioning, health perceptions, and vitality, as measured by the CFQ-R. The breadth of improvements reflects the systemic mechanism of action of ivacaftor compared to other therapies. Findings support the patient-reported value of ivacaftor treatment in this patient population.

http://dx.doi.org/10.1186/s12955-015-0293-6

### See also

Health Qual Life Outcomes. 2015 Jul 2;13:93. doi: 10.1186/s12955-015-0293-6.

### Keywords

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