primary studies - published RCT

Safety, efficacy and convenience of colistimethate sodium dry powder for inhalation (Colobreathe DPI) in patients with cystic fibrosis: a randomised study.

Code: PM23135343â€Ž **Year:** 2012 **Date:** 2015 **Author:** Schuster 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.

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See also

Thorax. 2012 Nov 7.

Keywords

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