primary studies - published RCT

Inhaled Hypertonic Saline in Infants and Children Younger Than 6 Years With Cystic Fibrosis: The ISIS Randomized Controlled Trial.

Code: PM22610452 **Year:** 2012 **Date:** 2015 **Author:** Rosenfeld M

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

data from Phase 3 studies (STRIVE/ENVISION) - post-hoc analysis

Participants

CF patients (nâ€‰=â€‰209)

Interventions

Post-ho analysis of patients (nâ€‰=â€‰209) who received 48 weeks of ivacaftor or placebo. Patients were assigned to tertiles according to FEV(1) response.

Outcome measures

FEV(1), sweat chloride, weight, CFQ-R, and pulmonary exacerbation

Main results

The number needed to treat (NNT) was calculated for specific thresholds for each outcome. Across all tertiles, numerical improvements in FEV(1), sweat chloride, CFQ-R and the frequency of pulmonary exacerbations were observed in ivacaftor-treated patients: the treatment difference versus placebo was statistically significant for all outcomes in the upper tertile and for some outcomes in the lower and middle tertiles. The NNT for aâ€‰â‰¥â€‰5% improvement in %predicted FEV(1) was 1.90, for aâ€‰â‰¥â€‰5% body weight increase was 5.74, and to prevent a pulmonary exacerbation was 3.85.

Authors' conclusions

This analysis suggests that the majority of patients with clinical characteristics similar to STRIVE/ENVISION patients have the potential to benefit from ivacaftor therapy.

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See also

JAMA. 2012 Jun 6;307(21):2269-77.

Keywords

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