primary studies - published RCT

Infant lung function tests as endpoints in the ISIS multicenter clinical trial in cystic fibrosis.

Code: PM26547590 **Year:** 2016 **Date:** 2016 **Author:** Davis SD

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

Secondary analysis of ISIS (a multicenter, randomized, double-blind, placebo-controlled trial) data

Participants

ISIS: Children aged 4 to 60 months with an established diagnosis of CF from 30 CF care centers in the United States and Canada. A total of 344 patients were assessed for eligibility; 321 participants were randomized; 29 (9%) withdrew prematurely.

Interventions

The active treatment group (n = 158) received 7% hypertonic saline and the control group (n = 163) received 0.9% isotonic saline, nebulized twice daily for 48 weeks. Both groups received albuterol or levalbuterol prior to each study drug dose.

Outcome measures

Secondary analysis of ISIS data was conducted in order to assess feasibility of infant pulmonary function tests (iPFTs) measures and their associations with respiratory symptoms. Standard deviations were calculated to aid in power calculations for future clinical trials.

Main results

Seventy-three participants enrolled, 70 returned for the final visit; 62 (89%) and 45 (64%) had acceptable paired functional residual capacity (FRC) and raised volume measurements, respectively. Mean baseline FEV0.5, FEF75 and FRC z-scores were 0.3 (SD: 1.2), -0.2 (SD: 2.0), and 1.8 (SD: 2.0).

Authors' conclusions

iPFTs are not appropriate primary endpoints for multicenter clinical trials due to challenges of obtaining acceptable data and near-normal average raised volume measurements. Raised volume measures have potential to serve as secondary endpoints in future clinical CF trials.

http://dx.doi.org/10.1016/j.jcf.2015.10.007

See also

J Cyst Fibros. 2016 May;15(3):386-91. doi: 10.1016/j.jcf.2015.10.007. Epub 2015 Nov 4.

Keywords

Child; hydration; Hypertonic Solutions; pharmacological_intervention; Airway clearance drugs -expectorants- mucolytic- mucociliary-; Respiratory System Agents;