primary studies - published RCT

A randomized controlled trial to evaluate the lung clearance index as an outcome measure for early phase studies in patients with cystic fibrosis.

Code: PM26856191 **Year:** 2016 **Date:** 2016 **Author:** Amin R

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

Randomized controlled cross-over trial

Participants

CF patients. 21 patients were randomized. 16 completed all study visits and all time point measurements. 18 patients contributed to the intention to treat analysis.

Interventions

Patients received inhalation of HS and isotonic saline (IS).

Outcome measures

Multiple breath washout (MBW) and spirometry were performed at 5 time points over 24 h. LCI was measured using both a nitrogen washout technique (LCIN2) and sulfur hexafluoride as a tracer gas (LCISF6). The primary endpoint was the change in the LCIN2 between baseline and 24 h. Secondary endpoints included change in LCISF6 and spirometry outcomes.

Main results

Twenty-one patients were randomized. Sixteen completed all study visits and all time point measurements. Eighteen patients contributed to the intention to treat analysis. Significant changes were not detected for either LCI or the spirometry outcomes. However, the primary outcome parameter (change in LCI between the baseline visits and 24 h after inhalation) demonstrated a trend towards improved LCI, in the HS treatment arm compared with the IS treatment arm, -0.60 LCIN2 (SE 0.32), p = 0.08); similar trends were not observed for spirometric measures. The overall effect size of HS was smaller than in previous studies of longer duration.

Authors' conclusions

These data suggest that LCI may potentially be used as an outcome measure in early phase trials with therapeutic agents that have a larger treatment effects than a single inhalation of HS.

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See also

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Keywords

Adolescent; Child; hydration; Hypertonic Solutions; Inhalation OR nebulised; nebuliser; non pharmacological intervention - devices OR physiotherapy; pharmacological_intervention; Sodium Chloride; Airway clearance drugs -expectorants- mucolytic- mucociliary-; Respiratory System Agents;