primary studies - published RCT

A CFTR corrector (lumacaftor) and a CFTR potentiator (ivacaftor) for treatment of patients with cystic fibrosis who have a phe508del CFTR mutation: a phase 2 randomised controlled trial.

Code: PM24973281 **Year:** 2014 **Date:** 2019 **Author:** Boyle MP

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

Randomised, double-blind, placebo-controlled trial

Participants

25 cystic fibrosis centres in Canada and the USA. Eligible participants were aged 36-72 months; had a confirmed diagnosis of cystic fibrosis; were able to comply with medication use, study visits, and study procedures; and were able to complete at least two technically acceptable trials of multiple breath washout (MBW).

Interventions

Participants were randomly assigned (1:1) via a web-based data entry system that confirmed enrolment eligibility to inhaled 7% hypertonic saline or 0.9% isotonic saline nebulised twice daily (for no more than 15 min per dose) for 48 weeks.

Outcome measures

The primary endpoint was the change in the LCI2.5 measured by nitrogen MBW from baseline to week 48.

Main results

Between April 21, 2015, and Aug 4, 2017, 150 participants were enrolled and randomly assigned, 76 to the hypertonic saline group and 74 to the isotonic saline group. Overall 89% of the MBW tests produced acceptable data. At 48 weeks, treatment with hypertonic saline was associated with a significant decrease (ie, improvement) in LCI2.5 compared with isotonic saline (mean treatment effect -0.63 LCI2.5 units [95% CI -1.10 to -0.15]; p=0.010). Six participants in the hypertonic saline group had ten serious adverse events and eight participants in the isotonic saline group had nine serious adverse events. The serious adverse events reported were cough (two patients [3%] in the hypertonic saline group vs three [4%] in the isotonic saline group), gastrostomy tube placement or rupture (two [3%] vs one [1%]), upper gastrointestinal disorders (one [1%] vs two [3%]), distal intestinal obstruction syndrome (one [1%] vs one [1%]), and decreased pulmonary function (none vs one [1%]). None of these serious adverse events was judged to be treatment related.

Authors' conclusions

Inhaled hypertonic saline improved the LCI2.5 in children aged 3-6 years, and could be a suitable early intervention in cystic fibrosis.

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See also

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Keywords

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