

primary studies - published RCT

Lumacaftor-Ivacaftor in Patients with Cystic Fibrosis Homozygous for Phe508del CFTR.

Code: PM25981758

Year: 2015 Date: 2019

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

Randomised crossover trial.

Participants

Twenty-two outpatients with CF from seven Centres

Interventions

Combining nebulised hyaluronic acid plus hypertonic saline (HA+HS) with oscillatory positive expiratory pressure (oscillatory-PEP). Usual care (HA+HS followed by autogenic drainage) and combined therapy (HA+HS with oscillatory-PEP followed by autogenic drainage). Each treatment was performed for 5 days.

Outcome measures

MAIN OUTCOME MEASURES: Sputum expectoration was measured during the nebulisation period (primary outcome), during autogenic drainage and for 24hours post intervention. The Cough and Sputum Assessment Questionnaire (CASA-Q) and its domains (cough symptoms, cough impact, sputum symptoms and sputum impact), the Leicester Cough Questionnaire (LCQ) and lung function tests were used. Tolerance and patient preference were registered.

Main results

22 participants [mean age 25 (standard deviation 8) years, percentage predicted forced expiratory volume in 1second 67 (22)] were recruited. Combined therapy promoted greater sputum expectoration than usual care during the nebulisation period {median difference 1.8ml [95% confidence interval (CI) 0.2-6.2]}. Both treatments led to similar expectoration during autogenic drainage and for 24hours post intervention. Combined therapy led to a greater improvement in the sputum symptoms domain [6.7 points (95% CI 3.3-13.3) and total CASA-Q score [2.4 points (95% CI 0.1-9.3)] compared with usual care. No differences in LCQ score or lung function were observed. Fewer adverse events were reported using combined therapy, which was selected as the preferred intervention.

Authors' conclusions

Combined nebulisation increased immediate sputum expectoration, improved sputum symptoms and reduced adverse events compared with usual care in patients with CF.

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See also

N Engl J Med. 2015 May 17.

Keywords

hydration; Hypertonic Solutions; Inhalation OR nebulised; pharmacological_intervention; Airway clearance drugs -expectorants-mucolytic- mucociliary-; Airway clearance technique; non pharmacological intervention - devices OR physiotherapy; Respiratory System Agents; Acapella; oscillating devices; Chest physiotherapy; Active Cycle of Breathing Technique -ACBT-;