primary studies - published RCT

Withdrawal of dornase alfa increases ventilation inhomogeneity in children with cystic fibrosis.

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

Single-centre, randomised, controlled, parallel group study

Participants

Patients with mild cystic fibrosis (CF) lung disease. 5-18 years old children with CF.

Interventions

Standard CF respiratory therapy. Dornase alpha

Outcome measures

Outcome measures were assessed at two visits one month apart. Primary outcome was absolute change in LCI. Secondary outcomes were FEV(1), FEF(25-75) and CF Questionnaire-revised (CFQ-R) respiratory symptom score. Possible harmful effects were assessed by comparing the occurrence of pulmonary exacerbations between groups.

Main results

28 children (median age 10.4 [interquartile range: 7.6; 13.5] years) with CF received standard care (n = 14) or intervention (n = 14). Compared with the control group, LCI increased (worsened) 1.74 (95% confidence interval: 0.62; 2.86) during withdrawal of dornase alfa, while FEV(1) (-6.8% predicted) and FEF(25-75) (-13.1% predicted) decreased significantly. Change in CFQ-R respiratory symptom score and the occurrence of pulmonary exacerbations did not differ significantly between groups.

Authors' conclusions

One month's withdrawal of dornase alfa caused increasing ventilation inhomogeneity and deteriorating FEV(1) and FEF(25-75) in school-age children with mild CF. Hence, adherence to dornase alfa optimally needs to be addressed when using LCI and spirometric parameters as endpoints, even in short-term clinical trials.

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See also

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Keywords

Adolescent; Bacterial Infections; Burkholderia cepacia; Child; Deoxyribonuclease; Infection; Inhalation OR nebulised; non pharmacological intervention - devices OR physiotherapy; pharmacological_intervention; Recombinant Proteins; Respiratory Tract Infections; Ventilators; Airway clearance drugs -expectorants- mucolytic- mucociliary-; Respiratory System Agents; Respiratory Tract Diseases; Dornase alpha; Pulmozyme;