

primary studies - published RCT

Liposome-mediated CFTR gene transfer to the nasal epithelium of patients with cystic fibrosis Comment in: Nat Med 1995 Jan;1(1):15-7.

Code: PM7584951

Year: 1995 **Date:** 1999

Author: Caplen NJ

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

randomized, double-blind, placebo-controlled n-of-1 assessment protocol

Participants

89 4-week assessments in 52 patients were reported. Twenty-four patients have completed the assessment process (12 responders and 12 nonresponders) to date.

Interventions

Nebulized recombinant human deoxyribonuclease (DNase). Patients underwent a maximum of three 4-week assessment periods (2 weeks saline, 2 weeks DNase each).

Outcome measures

Measurements performed at hospital (exercise, oximetry and spirometry) and home (symptom scores) were used to derive a scoring system

Main results

Forced expiratory volume in one second (FEV1) was the best discriminator of response, rising by >200 mL after DNase in 33 of 89 (37%) assessments compared with 3 of 89 (3%) after saline

<http://dx.doi.org/10.1038/nm0195-39>

See also

Nat Med. 1995 Jan;1(1):39-46.

Keywords

Adolescent; Adult; Deoxyribonuclease; Airway clearance drugs -expectorants- mucolytic- mucociliary-; pharmacological_intervention; Recombinant Proteins; Respiratory System Agents; Dornase alpha; Pulmozyme; Inhalation OR nebulised; nebuliser;