primary studies - published RCT

A placebo-controlled study of liposome-mediated gene transfer to the nasal epithelium of patients with cystic fibrosis.

Code: PM9135733 **Year:** 1997 **Date:** 1997 **Author:** Gill DR

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

double-blinded, placebo-controlled, clinical study

Participants

12 CF patients: 8 received treatment, 4 received placebo

Interventions

Cationic liposomes complexed with plasmid containing the human CFTR cDNA were administered to patients

Outcome measures

Biopsies of the nasal epithelium taken 7 days after dosing were normal

Main results

No significant changes in clinical parameters were observed. Functional expression of CFTR assessed by in vivo nasal potential difference measurements showed transient correction of the CF chloride transport abnormality in two patients (15 days after dosing in one patient). Fluorescence microscopy demonstrated CFTR function ex vivo. In cells from nasal brushings. In total, evidence of functional CFTR gene transfer was obtained in six out of the eight treated patients

Authors' conclusions

These results provide proof of concept for liposome-mediated CF gene transfer.

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See also

Gene therapy YR: 1997 VL: 4 NO: 3

Keywords

Adolescent; Adult; Gene Transfer Techniques; non pharmacological intervention - genetic& reprod; pharmacological_intervention; placebo;