primary studies - published RCT

Evidence for safety and efficacy of DOTAP cationic liposome mediated CFTR gene transfer to the nasal epithelium of patients with cystic fibrosis.

Code: PM9135734 **Year:** 1997 **Date:** 1997 **Author:** Porteous DJ

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

randomised, double-blinded study

Participants

8 CF patients (treatment group), 8 CF patients (placebo group)

Interventions

A single dose of 400 micrograms pCMV-CFTR:2.4 mg DOTAP or buffer only as administered to the nasal epithelium of patients

Outcome measures

Patients were monitored for signs and symptoms for 2 weeks before treatment and 4 weeks after treatment. Inflammatory cells were quantified in a nasal biopsy taken 3 days after treatment.

Main results

There was no evidence for excess nasal inflammation, circulating inflammatory markers or other adverse events ascribable to active treatment. Gene transfer and expression were assayed by the polymerase chain reaction. Transgene DNA was detected in seven of the eight treated patients up to 28 days after treatment and vector derived CFTR mRNA in two of the seven patients at +3 and +7 days. Transepithelial ion transport was assayed before and after treatment by nasal potential difference during drug perfusion and by SPQ fluorescence halide ion conductance. Partial, sustained correction of CFTR-related functional changes toward normal values were detected in two treated patients. The level of gene transfer and functional correction were comparable to those reported previously using adenoviral vectors or another DNA-liposome complex, but here were sustained and uncompromised by false positives.

Authors' conclusions

These results justify further studies with pCMV-CFTR-DOTAP aimed at treating CF lung disease.

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See also

Gene therapy YR: 1997 VL: 4 NO: 3

Keywords

Adult; Gene Transfer Techniques; non pharmacological intervention - diet; non pharmacological intervention - genetic& reprod; pharmacological_intervention; Supplementation;