

primary studies - published RCT

## **Efficacy of aerosolized tobramycin in patients with cystic fibrosis.**

**Code:** PM8497284

**Year:** 1993 **Date:** 1997

**Author:** Ramsey BW

### **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.

<http://dx.doi.org/10.1056/NEJM199306173282403>

### **See also**

The New England journal of medicine YR: 1993 VL: 328 NO: 24

### **Keywords**

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