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primary studies - published RCT

## **Cationic lipid-mediated CFTR gene transfer to the lungs and nose of patients with cystic fibrosis: a double-blind placebo-controlled trial.**

**Code:** PM10459902

**Year:** 1999

**Date:** 1999

**Author:** Alton EW

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

Double-blind randomised placebo controlled trial, parallel design

### **Participants**

Mean age 26.9 years. Confirmed CF, FEV1 >70%, sterile. 16 participants (all males).

### **Interventions**

Single dose of CFTR DNA+liposome, or liposome alone nebulised to lungs.

### **Outcome measures**

Adverse events, gene expression, CFTR protein expression, airway potential difference.

### **Main results**

Seven of the eight patients receiving the active complex reported mild influenza-like symptoms that resolved within 36 h. Six of eight patients in both the active and placebo groups reported mild airway symptoms over a period of 12 h following pulmonary administration. No specific treatment was required for either event. Pulmonary administration resulted in a significant (p

### **Authors' conclusions**

Cationic-lipid-mediated CFTR gene transfer can significantly influence the underlying chloride defect in the lungs of patients with cystic fibrosis.

[http://dx.doi.org/10.1016/S0140-6736\(98\)06532-5](http://dx.doi.org/10.1016/S0140-6736(98)06532-5)

### **See also**

Lancet. 1999 Mar 20;353(9157):947-54.

### **Keywords**

Adult; Gene Transfer Techniques; Inhalation OR nebulised; nebuliser; non pharmacological intervention - devices OR physiotherapy; non pharmacological intervention - diet; non pharmacological intervention - genetic& reprod; pharmacological\_intervention; placebo; Supplementation;