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.

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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;