primary studies - published RCT

Repeat administration of DNA/liposomes to the nasal epithelium of patients with cystic fibrosis.

Code: PM10918483 **Year:** 2000 **Date:** 2000 **Author:** Hyde SC

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

double-blinded study

Participants

10 subjects (treatment), 2 subjects (placebo).

Interventions

plasmid DNA expressing the CFTR cDNA complexed with DC-Chol/DOPE cationic liposomes or placebo. Each subject received three doses, administered 4 weeks apart.

Outcome measures

inflammation, toxicity or an immune response. Nasal epithelial cells were collected 4 days after each dose for a series of efficacy assays including quantitation of vector-specific DNA and mRNA, immunohistochemistry of CFTR protein, bacterial adherence, and detection of halide efflux ex vivo. Airway ion transport was also assessed in vivo by repeated nasal potential difference (PD) measurements.

Main results

There was no evidence of inflammation, toxicity or an immune response towards the DNA/liposomes or the expressed CFTR. On average, six of the treated subjects were positive for CFTR gene transfer after each dose. All subjects positive for CFTR function were also positive for plasmid DNA, plasmid-derived mRNA and CFTR protein.

Authors' conclusions

The efficacy measures suggest that unlike high doses of recombinant adenoviral vectors, DNA/liposomes can be successfully re-administered without apparent loss of efficacy.

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See also

Gene Ther. 2000 Jul;7(13):1156-65.

Keywords

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