primary studies - published, non RCT

Safety and biological efficacy of a lipid-CFTR complex for gene transfer in the nasal epithelium of adult patients with cystic fibrosis.

Code: PM10933918 **Year:** 2000 **Date:** 2000 **Author:** Noone PG

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

double-blind, placebo-controlled, rising-dose tolerance study

Participants

11 adult CF patients were studied

Interventions

cationic liposome [p-ethyl-dimyristoylphosphadityl choline (EDMPC) cholesterol] complexed with an expression plasmid containing hCFTR cDNA and placebo were sprayed into alternate nostrils at intervals over 7 h.

Outcome measures

safety and efficacy

Main results

After dosing, vector-specific DNA was present in nasal lavage of all subjects for up to 10 days. There were no adverse events. The vector-treated epithelium did not exhibit a significant increase in CFTR-mediated Cl- conductance from baseline and was not different from the placebo-treated nostril: mean deltaCFTR Cl- conductance, mV +/- SEM, -1.6+/-0.4 vs -0.6+/-0.4, respectively. CFTR-mediated Cl- conductance increased toward normal during repetitive nasal potential difference measurements over the 3 days before dosing which influenced the postdosing calculations. No vector-specific mRNA was detected in the nasal epithelial scrape biopsies, although endogenous CFTR mRNA was detected in all subjects.

Authors' conclusions

lipid-DNA complex is safe, but did not produce consistent evidence of gene transfer to the nasal epithelium by physiologic or molecular measures.

http://www.mrw.interscience.wiley.com/cochrane/clcentral/articles/907/CN-00298907/frame.html

See also

Molecular therapy : the journal of the American Society of Gene Therapy YR: 2000 VL: 1 NO: 1

Keywords

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