primary studies - published RCT

Liposome-mediated CFTR gene transfer to the nasal epithelium of patients with cystic fibrosis Comment in: Nat Med 1995 Jan;1(1):15-7.

Code: PM7584951 **Year:** 1995 **Date:** 1995 **Author:** Caplen NJ

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

double-blind, placebo-controlled trial

Participants

9 cystic fibrosis (CF) subjects (treatment group), 6 CF subjects (placebo group)

Interventions

active treatment: cationic liposome complexed with a complementary DNA encoding the CF transmembrane conductance regulator (CFTR); palcebo: liposome to the nasal epithelium.

Outcome measures

adverse clinical effects, restoration of the deficit

Main results

No adverse clinical effects were seen and nasal biopsies showed no histological or immuno-histological changes. A partial restoration of the deficit between CF and non-CF subjects of 20% was seen for the response to low Cl- perfusion following CFTR cDNA administration. This was maximal around day three and had reverted to pretreatment values by day seven. In some cases the response to low Cl- was within the range for non-CF subjects. Plasmid DNA and transgene-derived RNA were detected in the majority of treated subjects.

Authors' conclusions

Although these data are encouraging, it is likely that transfection efficiency and the duration of expression will need to be increased for therapeutic benefit.

http://dx.doi.org/10.1038/nm0195-39

See also

Nat Med. 1995 Jan;1(1):39-46.

Keywords

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