
primary studies - published RCT

Comparison of DNA-lipid complexes and DNA alone for gene transfer to cystic fibrosis airway epithelia in vivo.

Code: PM9294121

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

randomized, double-blind study.

Interventions

Complexes of DNA-lipid were administered to one nostril and DNA alone to the other nostril

Outcome measures

Electrophysiologic measurements, measurements of vector-specific CFTR transcripts

Main results

Electrophysiologic measurements showed that DNA-lipid complexes partially corrected the Cl⁻ transport defect. Importantly, the pCF1-CFTR plasmid alone was at least as effective as complexes of DNA with lipid. Measurements of vector-specific CFTR transcripts also showed gene transfer with both DNA-lipid and DNA alone

Authors' conclusions

These results indicate that nonviral vectors can transfer CFTR cDNA to airway epithelia and at least partially restore the Cl⁻ transport defect characteristic of CF. However, improvements in the overall efficacy of gene transfer are required to develop a treatment for CF.

<http://dx.doi.org/10.1172/JCI119676>

See also

J Clin Invest. 1997 Sep 15;100(6):1529-37.

Keywords

Adolescent; Adult; Amiloride; Gene Transfer Techniques; non pharmacological intervention - genetic& reprod; pharmacological_intervention; Terbutaline; Airway clearance drugs -expectorants- mucolytic- mucociliary-; ENaC antagonists - Sodium Channel Blockers; Respiratory System Agents;