

primary studies - published RCT

Evidence for safety and efficacy of DOTAP cationic liposome mediated CFTR gene transfer to the nasal epithelium of patients with cystic fibrosis.

Code: PM9135734

Year: 1997 **Date:** 2001

Author: Porteous DJ

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

RCT

Participants

520 patients, aged > or = 6 years, with moderate-to-severe CF

Interventions

Patients received tobramycin solution for inhalation (TSI) or placebo, which was administered in alternating cycles of 28-days-on and 28- days-off therapy, plus their usual CF care for 6 months with open-label follow-up extended to 2 years

Outcome measures

long-term safety and effectiveness

Main results

Most AEs declined in frequency with increasing TSI exposure. Patients receiving TSI spent 25 to 33% fewer days in the hospital. Following the initiation of TSI treatment, patients experienced significant increases in FEV(1). FEV(1) values were maintained above baseline for the duration of the study series. Antibiotic susceptibility of the bacterial isolates did not predict clinical response.

Authors' conclusions

TSI was safe, well-tolerated, and effective for long-term treatment (96 weeks) of P aeruginosa colonization and infection in CF patients.

<http://dx.doi.org/10.1038/sj.gt.3300390>

See also

Gene therapy YR: 1997 VL: 4 NO: 3

Keywords

Adolescent; Adult; Aminoglycosides; Anti-Bacterial Agents; Bacterial Infections; Child; Infection; Inhalation OR nebulised; nebuliser; non pharmacological intervention - devices OR physiotherapy; pharmacological_intervention; Pseudomonas aeruginosa; Pseudomonas; Respiratory Tract Diseases; Respiratory Tract Infections; Tobramycin;