primary studies - published RCT

Gentamicin-induced correction of CFTR function in patients with cystic fibrosis and CFTR stop mutations.

Code: PM14534336 **Year:** 2003 **Date:** 2003 **Author:** Wilschanski M

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

double-blind, placebo-controlled, crossover trial

Participants

patients with stop mutations in CFTR or patients homozygous for the DeltaF508 mutation

Interventions

two drops containing gentamicin (0.3 percent, or 3 mg per milliliter) or placebo in each nostril three times daily for two consecutive periods of 14 days.

Outcome measures

Nasal potential difference was measured at base line and after each treatment period. Nasal epithelial cells were obtained before and after gentamicin treatment from patients carrying stop mutations, and the C-terminal of surface CFTR was stained.

Main results

Gentamicin treatment caused a significant reduction in basal potential difference in the 19 patients carrying stop mutations (from -45+/-8 to -34+/-11 mV, P=0.005) and a significant response to chloride-free isoproterenol solution (from 0+/-3.6 to -5+/-2.7 mV, P

Authors' conclusions

In patients with cystic fibrosis who have premature stop codons, gentamicin can cause translational "read through," resulting in the expression of full-length CFTR protein at the apical cell membrane, and thus can correct the typical electrophysiological abnormalities caused by CFTR dysfunction.

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See also

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Keywords

Adolescent; Adult; Anti-Bacterial Agents; Child; CFTR Modulators; Gentamicin; Intranasal; pharmacological_intervention; Bacterial Infections; Respiratory Tract Infections; Respiratory Tract Diseases; Infection; Aminoglycosides;