primary studies - published RCT

Evidence of CFTR function in cystic fibrosis after systemic administration of 4-phenylbutyrate.

Code: PM12095312 **Year:** 2002 **Date:** 2002 **Author:** Zeitlin PL

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

randomised, double-blind, placebo-controlled, dose-escalation and safety study

Participants

19 adults with CF (homozygous deltaF508)

Interventions

Three dose levels (20, 30, or 40 g divided t.i.d.) of drug or placebo were given for 1 week.

Outcome measures

Serial measurements of chloride transport by nasal potential difference (NPD) testing and metabolic safety testing were performed.

Main results

A maximum tolerated dose of 20 g was defined based on minimal adverse reactions, the safety profile, and a statistically significant induction of chloride transport that was maximal by day 3.

Authors' conclusions

This short-term phase I/II study demonstrates proof of principle that modulation of deltaF508 CFTR biosynthesis and trafficking is a viable therapeutic approach for cystic fibrosis.

http://dx.doi.org/10.1006/mthe.2002.0639

See also

Molecular therapy : the journal of the American Society of Gene Therapy YR: 2002 VL: 6 NO: 1

Keywords

4-phenylbutyrate; Adult; CFTR Modulators; pharmacological_intervention;