primary studies - published RCT

A pilot clinical trial of oral sodium 4-phenylbutyrate (Buphenyl) in deltaF508-homozygous cystic fibrosis patients: partial restoration of nasal epithelial CFTR function.

Code: PM9476862 **Year:** 1998 **Date:** 1998 **Author:** Rubenstein RC

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

randomized, double-blind, placebo-controlled trial

Participants

18 deltaF508-homozygous patients with CF

Interventions

maximum approved adult dose of 4PBA, 19 grams p.o. divided t.i.d., given for 1 wk

Outcome measures

Nasal potential difference (NPD) response patterns and sweat chloride concentrations were determined before and after study drug treatment, and 4PBA and metabolites were assayed in plasma and urine at the end of study drug treatment

Main results

Subjects in the 4PBA group demonstrated small, but statistically significant improvements of the NPD response to perfusion of an isoproterenol/amiloride/chloride-free solution; this measure reflects epithelial CFTR function and is highly discriminatory between patients with and without CF. Subjects who had received 4PBA did not demonstrate significantly reduced sweat chloride concentrations or alterations in the amiloride-sensitive NPD. Side effects due to drug therapy were minimal and comparable in the two groups.

Authors' conclusions

These data are consistent with 4PBA therapy inducing CFTR function in the nasal epithelia of deltaF508-homozygous CF patients.

http://ajrccm.atsjournals.org/content/157/2/484.full.pdf

See also

Am J Respir Crit Care Med. 1998 Feb;157(2):484-90.

Keywords

4-phenylbutyrate; Adolescent; Adult; Buphenyl; CFTR Modulators; Genetic Predisposition to Disease; Oral; pharmacological_intervention;