primary studies - published RCT

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

Code: PM12095312 **Year:** 2002 **Date:** 2005 **Author:** Zeitlin PL

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

1) short-term RCT (12 week) trial; 2) long-term RCT (9 months)

Participants

16 CF children and adults. 12 completed the 9-month trial.

Interventions

1) cyproheptadine hydrochloride (CH) for 12 weeks; 2) All patients receiving placebo in the original short-term study received CH 4 mg up to four times a day in the long-term study continuation, and those receiving CH in the short-term study continued on the drug. No pill counts were done, and patients were queried at quarterly visits as to their CH use.

Outcome measures

1) weight 2) Anthropometrics and spirometry were also done quarterly, and antibiotic use was quantified.

Main results

1) good results compared to placebo 2) Subjects who had changed from placebo (CH2 group) gained weight significantly over 3-6 months, and those continuing on CH (CH1 group) generally maintained previously gained weight over the duration of the study. Select spirometric measures improved in both groups but not significantly, and side effects were mild.

Authors' conclusions

CH appears to be an effective appetite stimulant in CF, and generally maintains its effect over time with an acceptable side-effect profile.

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See also

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

Keywords

Adolescent; Appetite Stimulants; Cyproheptadine; Gastrointestinal Agents; pharmacological_intervention; Malnutrition; Nutrition Disorders;