primary studies - published RCT

Assessing the usefulness of outcomes measured in a cystic fibrosis treatment trial.

Code: PM16806875 **Year:** 2007 **Date:** 2011 **Author:** Suri R

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

phase II randomized, investigator-blinded, parallel-group pilot study

Participants

16 infants with CF and PI, 6 to 30 months of age

Interventions

The study design included a run-in period (days 1-5) and an experimental period (days 6-11). Pancrelipase microtablets (2-mm, enteric coated) were provided orally. 500 U lipase/kg/meal for 5 days (baseline period). Subsequently, subjects were randomly assigned to 1 of 4 treatment groups (each n = 4), receiving 500, 1000, 1500, or 2000 U (Ph. EUR) of lipase/kg/meal, respectively, for 5 days (experimental period).

Outcome measures

The primary endpoint was medication efficacy assessed by the 72-hour fecal fat excretion, expressed as coefficient of fecal fat absorption (CFA), and 13C mixed triglyceride breath test. Secondary endpoints were safety and palatability.

Main results

Overall compliance, defined as used study medication, was 89% to 99% for the entire study. None of the 4 dose regimens significantly influenced the CFA, relative to the baseline period (median range 83%-93%). During the run-in period the median cumulative % 13C was 11 (range -8 to 59). After randomization the median cumulative % 13C was 18 (range 14-23) in the 500-U, 14 (range -1 to 17) in the 1000-U, 10 (range 10-27) in the 1500-U, and 3 (range 1-49) in the 2000-U groups. Palatability was scored fair to good by the parents in each of the treatment groups. Gastrointestinal symptoms were reported in some patients, including common adverse events reported in clinical trials involving pancreatic enzyme therapy. No serious or other adverse events were reported.

Authors' conclusions

Treatment with Pancrease MT at a dosage of 500 U lipase/kg/meal resulted in a CFA of approximately 89% in pediatric subjects ages 6 to 30 months with PI resulting from CF. Pancrease MT doses were well tolerated and mean palatability was scored as fair to good. Present results do not indicate that a dosage higher than 500 U (Ph. EUR) lipase/kg/meal increases the coefficient of fat absorption in a cohort of infants 6 to 30 months of age.

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See also

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Keywords

child; infant; Pancreas insufficiency; Pancreatic Diseases; Gastrointestinal Diseases; Malabsorption; Nutrition Disorders; Pancreatic Enzyme Replacement Therapy; Enteric-Coated; Microtablets; Oral; pharmacological_intervention; Gastrointestinal Agents; Pancrelipase;