

Pancreatic Enzyme Therapy and Clinical Outcomes in Patients with Cystic Fibrosis

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Objective To assess the relationship between pancreatic enzyme therapy (PET) and the clinical outcomes of growth, abdominal pain, constipation, gassiness, and number of stools in cystic fibrosis (CF).

Study design Patients (n = 1215) >4 weeks of age from 33 Cystic Fibrosis Foundation accredited sites who had a sweat chloride >60 mmol/L or two CF-causing mutations were enrolled using a proportionate sampling strategy in a nonblinded study. Patients submitted a stool sample and completed a questionnaire. The study coordinator also completed a questionnaire for each patient. Enzyme dosing and growth, abdominal pain, gassiness, constipation, and number of stools were compared.

Results Of the 1215 enrolled patients, 1131 (93.1%) were prescribed PET. Only 14.9% had pancreatic function assessed before enrolling in this study. Stool elastase-1 analysis identified 1074 (89%) patients as pancreatic insufficient (PI). There was no association between PET and the outcomes: growth, abdominal pain, gassiness, constipation, and number of stools.

Conclusion PET dose is not correlated with growth or gastrointestinal symptoms. More sensitive outcome measures of the effectiveness of PET in patients with CF are needed to guide treatment of PI.