primary studies - published RCT

Liprotamase long-term safety and support of nutritional status in pancreatic-insufficient cystic fibrosis.

Code: PM22266487 **Year:** 2012 **Date:** 2012 **Author:** Borowitz D

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

phase III 12-month open-label RCT

Participants

Patients 7 years and older with cystic fibrosis (CF) who have exocrine pancreatic insufficiency (EPI). A total of 215 subjects were enrolled and 214 received at least 1 dose of liprotamase (mean 5.5 capsules per day).

Interventions

Pancreatic enzyme replacement therapy (PERT). Liprotamase, a nonporcine, highly purified biotechnology-derived. All of the patients were required to discontinue their long-term use of porcine PERTs at the time of enrollment. Dosing started at 1 capsule of liprotamase (32,500 US Pharmacopoeia (USP) units crystallized cross-linked lipase, 25,000 USP units crystallized protease, and 3,750 USP units amorphous amylase) per meal or snack; dose could be increased based on protocol-defined parameters.

Outcome measures

Nutrition and age-appropriate growth and weight gain. Height, weight, and body mass index z scores. Lung function as measured by forced expiratory volume in 1 second. Laboratory tests, including levels of fat-soluble vitamins. Tolerability and safety. Adverse events, primarily gastrointestinal

Main results

A total of 215 subjects were enrolled and 214 received at least 1 dose of liprotamase (mean 5.5 capsules per day). During the study period, height, weight, and body mass index z scores and lung function as measured by forced expiratory volume in 1 second were stable. There were no clinically meaningful changes in laboratory tests, including levels of fat-soluble vitamins. Liprotamase was well tolerated without any significant safety concerns. Adverse events, primarily gastrointestinal, led to treatment discontinuation for 36 subjects (16.8%), most within the first 3 months.

Authors' conclusions

Treatment with a mean of 5.5 capsules of liprotamase per day, during meals and snacks, for up to 12 months was safe, well tolerated, and associated with age-appropriate growth and weight gain or weight maintenance in subjects with CF-related EPI.

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See also

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Keywords

Gastrointestinal Diseases; Liprotamase; pharmacological_intervention; Pancreas insufficiency; Pancreatic Diseases; Pancreatic Enzyme Replacement Therapy; Malabsorption; Nutrition Disorders; Gastrointestinal Agents;