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Safety and efficacy of a novel microbial lipase in patients with exocrine pancreatic insufficiency due to cystic fibrosis: A randomized controlled clinical trial

To evaluate the safety and efficacy of a novel microbial lipase (NM-BL) in a liquid formulation for the treatment of exocrine pancreatic insufficiency in patients with cystic fibrosis, we performed a phase IIa proof-of-concept study. We conducted a double-blind, randomized, placebo controlled crossover study in patients with cystic fibrosis (CF) and exocrine pancreatic insufficiency (EPI). Adolescent and adult patients with CF were randomized to receive NM-BL or placebo for 1 week as replacement for their usual pancreatic enzyme formulation. They were subsequently crossed-over to the alternate study treatment. The coefficient of fat absorption (CFA) was evaluated as primary end point. Symptoms and adverse events were evaluated as secondary end points. Thirty-one patients were randomized in the study and 22 patients completed both treatment periods. During treatment with NM-BL, the CFA was significantly higher (72.7%) compared to placebo (53.8%) with a difference between groups of 18.8% (P<0.001). Subjective assessment of stool fat and stool consistency also improved under treatment with NM-BL. Adverse events was mostly gastrointestinal in nature and consistent with the underlying disease. Currently available pancreatic enzyme products are limited because of the lack of liquid formulations and being largely porcine based. The novel microbial lipase NM-BL was safe and effective in this short term trial. The trial provided clinical proof-of-concept for this novel microbial lipase as a treatment for EPI in CF.

Biography

James E Heubi, MD is an Associate Dean for Clinical and Translational Research; Professor of Pediatrics at the University of Cincinnati College of Medicine. He is the Director of the Center for Clinical and Translational Science and Training which is the vehicle by which the University of Cincinnati, Cincinnati Children's Hospital and the Veterans Administration Medical Center administer the NIH-funded, Clinical Translational Science Award. He has been an active Clinical Investigator with over 200 peer reviewed publications and was the long term Program Director for the NIH funded General Clinical Research Center. He is the Principal Investigator and Co-Investigator on NIH grants and participates is a local principal or co-investigator on 2 rare disease networks supported by the NIH. His research interests include cholestatic liver disease in childhood and its complications, inborn errors of bile acid metabolism, bone disease in gastrointestinal and hepatobiliary disorders, fat absorption, and cholesterol absorption and metabolism.

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