

Children's Hospital of Pittsburgh of UPMC Division of Genetic and Genomic Medicine is looking for patients 10 years of age or older with a diagnosis of Medium-chain acyl-CoA dehydrogenase (MCAD) deficiency to participate in a 7-week research study looking at the medication ACER-001 and its effects on fasting and reduction in blood glucose levels. Participants must have a confirmed diagnosis of MCAD deficiency based on DNA sequencing and be willing to come to Pittsburgh three or four times during the study. The study will involve a screening visit, an overnight admission to monitor fasting glucose levels and other metabolites for up to 32 hours, dosing of study medication for 4 weeks, a second overnight admission for up to 24 hours to monitor fasting glucose levels and other metabolites, and a final end-of-study visit. Participants will also receive multiple phone calls during the study to check on symptoms while taking the study medication. Participants will be asked to take the study medication 1 or 2 times daily. Travel and a participant stipend will be covered by the study site. If you are interested in learning more about this trial, please contact Elizabeth McCracken at 412-692-5662.