

Children's Hospital of Pittsburgh of UPMC Division of Genetic and Genomic Medicine is looking for patients 4 years of age or older with a diagnosis of Medium-chain acyl-CoA dehydrogenase (MCAD) deficiency to participate in a 4-week research study looking at the medication ACER-001 and its effects on blood glucose levels. This medication is FDA-approved for people with Urea Cycle Disorders but not for people with MCADD. Use of ACER-001 in this study is experimental. Participants must have a confirmed diagnosis of MCAD deficiency based on DNA sequencing and be willing to come to Pittsburgh three times during the study. The study will involve a screening visit. If participants are eligible following the screening visit, they will be given a continuous glucose monitor (CGM) to wear for 10 days. They will be asked to return to the site within 2 weeks of the screening visit. At that visit they will be given their first dose of ACER-001 followed by 8 hours of blood and urine collection. They will return home with instructions to take ACER-001 twice a day until they return for their final visit. They will also be given the CGM to wear for 10 days. The participant will be asked to return to the site approximately 11 days later for the final visit. Participants will also receive multiple phone calls during the study to check on symptoms while using the CGM and taking the study medication.

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.