UPMC | CHILDREN'S HOSPITAL OF PITTSBURGH

The UPMC Children's Hospital of Pittsburgh 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. This medication is FDA-approved for people with Urea Cycle Disorders, but not for patients with MCADD. The use of ACER-001 in this study is experimental.

We want to study its effects on blood sugar levels in people with MCAD. Participants must have a confirmed diagnosis of MCAD deficiency based on genetic testing and be willing to come to Pittsburgh for 3 visits. Each visit may take up to 10 hours to complete. Travel and a participant stipend will be covered by the study site.

Call the number below for more details about the study.

If you are interested in learning more about this trial, please contact Elizabeth McCracken at 412-692-5662.