

Open research study – now recruiting Triheptanoin (C7) in Glucose transporter type I deficiency (G1D)

Location: The University of Texas Southwestern Medical Center, Dallas, Texas.

Funding agencies: National Institute of Neurological Disorders and Stroke (National Institutes of Health, NIH) and Glut1 Deficiency Foundation

Oversight: UT Southwestern Medical Center Institutional Review Board, FDA and National Institutes of Health

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A research study is currently testing food-grade C7 oil in G1D. The purpose is to answer whether C7 impacts neuropsychological test results (cognitive capacity, including attention, comprehension, memory, etc., among many other aspects that impact school performance and potential for personal development and independence) in G1D patients taking a regular diet. EEG and blood work are also part of the study.

Patients diagnosed by DNA testing or by PET scan are eligible. They may not be taking a ketogenic diet. No patient will be asked to or should change diets just for this study.

The study lasts 9 months for each participant (6 months on C7 and then 3 months off C7). This usually requires three 2-day visits to Dallas over 9 months. About one of the 2 days day will be inpatient. Individual discussion about any general aspects of G1D is also available and free.

This is NIH-funded research. Therefore, there are no patient or medical insurance charges. Travel expenses up to \$1,220 plus a \$250 stipend for lost time (per visit) are available upon submission of expense receipts after each visit is completed. Reimbursement is via prepaid credit card. Discounted local hotel rates are available. Patient and family satisfaction with the logistics of the study has so far been exceptional. Many of them live in remote areas of North America or overseas.

Background: There are few effective treatments for G1D. Triheptanoin is a food-grade, medical food candidate that has been pioneered by researchers at UT Southwestern Medical Center for the treatment of G1D (see <https://www.ncbi.nlm.nih.gov/pubmed/25110966>). These researchers have recently determined the optimal dose for C7 in G1D patients. They have also determined, in many other laboratory research studies, the main brain biochemical defect in G1D and how C7 works in the G1D brain. All this work is part of a much larger long-term patient care and medical and laboratory research program unique in the U.S.

At the conclusion of the study, if the results are positive, it is expected that C7 will be ready for further testing or for designation as a medical food (such as vitamins or MCT oil). The investigators at UT Southwestern Medical Center are salaried, work on a nonprofit basis and have declined all financial, intellectual property or other competing interests that can bias medical research and inflate the cost of medical treatments.

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