

Upcoming research studies starting in 2017

Title: Treatment development of triheptanoin (C7) for Glucose transporter type I deficiency (G1D)

Study location: The University of Texas Southwestern Medical Center, Dallas, Texas.

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

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A series of new studies are being planned using food-grade C7 oil in G1D. The ultimate purpose of these studies using the proposed medical food C7 is to answer the question whether C7 impacts neuropsychological performance (cognitive capacity) in G1D patients taking either a regular diet or a ketogenic diet. The goal of the first study is to determine the maximum tolerated dose of C7 oil in G1D, which has not been established to date. Patients diagnosed by DNA testing or by PET scan will be eligible for this study. They may not be receiving a ketogenic diet. After completion of this first study, a second study will evaluate C7 impact on neuropsychological performance in G1D patients receiving a regular diet. A third study will assess compatibility between C7 and the ketogenic diet. No patient will be asked to or should change diets for any of these studies.

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>). First, an optimal dose for C7 has never been determined and this will be accomplished in the initial study. Second, until now, the researchers have focused their investigations on two indicators of patient improvement after C7 treatment: 1) EEG and 2) Neuropsychological testing scores. However, it is well known that absence epilepsy can be treated in terms of EEG improvement with little impact on neuropsychological performance or school attainment (<https://www.ncbi.nlm.nih.gov/pubmed/24089388>). In practical terms, this type of isolated EEG improvement (or lack thereof) means little to patient wellbeing and consequently has motivated the investigators to primarily focus on the effects of C7 on neuropsychological performance rather than on EEG (although EEG will also be studied). Third, there is concern that C7 may interfere with a ketogenic diet and so the investigators will study this potential compatibility or incompatibility rigorously. At the conclusion of these studies, it is expected that C7 may be ready for further testing as a medical food. The investigators at UT Southwestern Medical Center work on a non profit basis and have no financial or other competing interests that may bias or otherwise impact the neutrality of the results of these studies. Their goal is to answer the question whether C7 should eventually be made commercially available as a medical food in line with other widely available supplements such as vitamins or MCT oil.

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