



Section of
Experimental and Systems Pharmacology
COLLEGE OF PHARMACY

January 15, 2015

Dear SSADH patients and families:

I am writing to let you know about a research study for patients with succinic semialdehyde dehydrogenase enzyme (SSADH) deficiency at the National Institutes of Health (NIH). This study is being led by Dr. William Theodore, Clinical Epilepsy Section, NINDS, NIH. I am an associate investigator on the study. The study number at NIH is 14-N-0033.

The study will test whether a research drug called SGS-742 is helpful and safe to take. SGS-742 interferes with some of the actions of the brain chemical GABA. We will look at how SGS-742 affects brain activity and performance on tests of attention, thinking, and memory. We will also look at the effects of SGS-742 on brain chemistry. SGS-742 has not been given previously to people with SSADH. One research study of SGS-742 in adults with mild cognitive impairment found that the drug seemed to help memory and thinking.

Patients may qualify for this study if they are 8 years of age or older, have a diagnosis of SSADH and have symptoms and features of SSADH deficiency. Patients may not qualify for participation in this study if they drink more than a moderate amount of alcohol or if they use recreational drugs, are pregnant or breast feeding, or have a medical condition that could make participation unsafe. Participation lasts about 14 months.

Each participant in the study will take a capsule with SGS-742 for 6 months and placebo, a capsule without active drug in it, for another 6 months. Neither the participant nor the study doctors will know when the capsule contains SGS-742 and when it has placebo. Participants in the study will have electroencephalogram (EEG), magnetic resonance imaging (MRI), Transcranial Magnetic Stimulation (TMS), neuropsychological testing (tests of learning and memory), blood and urine tests, and optional lumbar puncture.

This study is being conducted as research and participation is voluntary. Costs associated with travel are covered for the patient and a companion (if necessary). There is no cost for participation in this study. For patients who are under the age of 18, a parent or guardian must grant permission to participate.

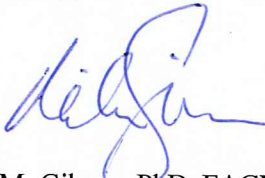
If you have any questions about study procedures or enrollment activities, please contact Ms. Tamika Mason, Recruitment Specialist:

Tamika N. Mason Patient Care and Recruitment Specialist	301-496-1923 Tamika.mason@nih.gov
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The study organizers (Dr. Theodore, Dr. Phillip Pearl) and I realize that the time commitment for this trial is significant and that you may have additional questions or concerns. The decision to enroll or not to enroll is a very personal one, and the study organizers fully respect individual needs and decisions.

If you have any questions, please contact our colleagues listed above, or feel free to contact myself (mike.gibson@wsu.edu) or Dr. Pearl (Phillip.pearl@childrens.harvard.edu). A description of this study is available at <http://www.clinicaltrials.gov>

Sincerely,



K. M. Gibson, PhD, FACMG
Allen I. White Distinguished Professor
Chair, Experimental and Systems Pharmacology

