UC San Diego
UC San Diego News Center

June 10, 2015 | By Scott LaFee

Clinical Trial Launched to Assess Safety and Efficacy of Autism Drug Treatment

UC San Diego researchers open first study of suramin in children with autism

Researchers at the University of California, San Diego School of Medicine have launched a clinical trial to investigate the safety and efficacy of an unprecedented drug therapy for autism.

The phase 1 clinical trial, which is recruiting 20 qualifying participants, will evaluate suramin – a century-old drug still used for African sleeping sickness – as a novel treatment for children with a diagnosis of Autism Spectrum Disorder (ASD). Previous published research by Robert K. Naviaux, MD, PhD, professor of medicine, pediatrics and pathology at UC San Diego School of Medicine, and colleagues reported that a single injection of suramin reversed symptoms of ASD in mouse models.

This trial is the first to test suramin in children with ASD.

“Autism affects 1 to 2 percent of children in the United States,” said Naviaux, co-director of the Mitochondrial and Metabolic Disease Center at UC San Diego. “Hundreds of genetic and environmental factors have been shown to increase the risk of ASD, but treatment options for the core symptoms are limited. To date, there are no medications that can cure ASD, and very few that can improve its core symptoms.”

To participate in the study, a child must be a boy between the ages of 4 - 17 years and have been diagnosed with ASD by a psychologist or physician. He must be a resident of San Diego County and cannot have a known genetic cause of mutation (such as Fragile X syndrome) or be taking any prescription medications. He cannot have been hospitalized within the last two months or be planning to begin any new medical treatments during the time of the study. The study will involve 10 to 12 clinical visits over three to four months for each child.

All testing and treatment will occur in San Diego County. Participants who complete the trial will be compensated for their time.

In the trial, suramin will be given as a single dose through an intravenous line. Half of the participating children will receive suramin; half will receive a placebo (saline infusion). Behavioral and medical tests will be conducted before and after treatment, and include some blood and urine analyses.

The trial is the first clinical investigation of a novel theory, advanced by Naviaux, that posits autism may be a consequence of abnormal cell communication resulting from abnormal activation of the cell danger response.

Cells threatened or damaged by microbes, such as viruses or bacteria, or by physical forces or by chemicals, such as pollutants, react defensively, a part of the normal immune response, Naviaux said. Their membranes stiffen. Internal metabolic processes are altered – most notably mitochondria, the cells' critical "power plants" – resulting in activation of the cell danger response and reduced communications between cells.

Naviaux said the cell danger response theory does not contradict other research regarding the causes of autism. Rather, it offers another perspective and, perhaps, a new therapeutic target.

Because suramin treatment for autism is unprecedented, Naviaux emphasized it is not known whether the drug will produce any beneficial effect in humans. He noted that suramin, as currently constituted, cannot be used for more than a few months without a risk of toxicity in humans and that it is not available as an ongoing treatment.

"So even if participants show benefit during the trial, it will not be possible to continue treatment after the study," said Naviaux. "However, if the study is successful, they may be eligible for the next suramin study."

For more information about the suramin trial, contact: Jeanne Townsend, PhD, jtownsend@ucsd.edu, 858-246-1932; Suzanne Goh, MD, sgoh@gohmd.com, 858-304-6440; or Alan Lincoln, PhD, MSCP, alincoln@alliant.edu, 858-444-8823. Please provide child's date of birth and gender in emails. Prior to trial enrollment, a clinician's report verifying autism diagnosis will be required.

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