

Naviaux Lab Cautionary Statement on Suramin

June 7, 2017

In response to inquiries about the results of our small SAT1 study that evaluated the African sleeping sickness (trypanosomiasis) medicine suramin as a potential autism treatment, we caution against the medicine's use outside of rigorously controlled, FDA- and IRB-authorized clinical trials at this time. See a description of the trial at <http://naviauxlab.ucsd.edu/science-item/autism-research/> and materials at <http://health.ucsd.edu/suramin>. Continued caution is required because:

1. **Safety Remains Unproven.** The Suramin Autism Treatment-1 (SAT1) trial was not designed to be a trial of benefit, but rather as a small pilot study of safety and activity. This study was designed to test the cell danger hypothesis, and to better understand the pharmacology and metabolomics of a completely new approach to the treatment of ASD. The low-dose of suramin used in the SAT1 trial was known to be safe in animal models of autism-like behavior, but the current results are the first in human autism. Low-dose suramin was found to be safe in this small study. High doses of suramin used to treat cancer carry a risk of toxicity. Cancer doses are 25 times greater than doses used in the SAT1 study. Suramin has been used safely in both children and adults at medium dosage for nearly 100 years to treat African sleeping sickness (trypanosomiasis). Further safety testing of low-dose suramin in ASD is needed.
2. **Outcomes are Unproven.** The SAT1 study involved just 5 children receiving the drug and another 5 receiving the placebo—a number too small to provide generalized conclusions applicable to the larger autism population. In addition, the occurrence of a self-limited and asymptomatic rash that lasted for 2-4 days then disappeared, created problems with keeping the study fully blinded. This occurred even though in some children the rash was so mild and about the size of a silver dollar that parents concluded the child had slept wrong on their pillow and did not ascribe the rash to suramin. The rash was not visible on exposed skin to behavioral examiners at any time during the study and parents were instructed not to discuss it with the behavioral team to avoid bias. In the end, a careful reader of our study will likely conclude the findings are either incorrect due to the small size of the study or the findings represent an important advance. Only future studies will tell us which conclusion is correct.

For these reasons, the Naviaux lab asserts that it is premature to make any broad conclusions about suramin's potential—or the potential of antipurinergic therapy in general—as an autism treatment. The next step is a larger Phase II clinical trial to examine safety and efficacy of several doses of suramin given over several months in a rigorously controlled and objective manner. The SAT2 study is currently in planning stages as we continue to raise financial support for the trial. If you would like to help, you can make a tax-free donation at <http://naviauxlab.ucsd.edu/support/>.