

USA Drug Safety Trial Update - 1st August 2017

Dear Friends,

It's a spectacular Saturday morning in Saint Louis as I write this to you. I always appreciate your continued support, encouragement, and kind words.

I have received many questions regarding our ongoing clinical trial, as well as questions related to our next step lately.

Although we don't have a concrete plan yet, we consider the following possibilities.

1. A longer duration, more participants, Multi-centre
2. Compare the efficacy of dantrolene sodium, valproic acid, and dantrolene sodium plus valproic acid

We are also developing breakthrough treatments for Wolfram syndrome as dantrolene and valproic acid are old drugs originally developed for other medical conditions.

Here are our new plans.

1. We have been developing a new drug designed for Wolfram syndrome (ER stress stabilizers) together with NIH/NCATS to delay/halt the progression of the disease. Pre-clinical studies in mouse models of Wolfram have been designed. We are seeking funds to complete these studies.
2. Regenerative therapies using a novel neurotrophic factor in combination with gene transfer technology for visual impairment have been designed. We are seeking funds for testing these new therapies in mouse models of Wolfram syndrome.

I think we are making progress, and need to speed up as we are racing against time. Thank you again for your support.

Take care,

Fumi Urano, MD