

Dear families and friends of the NPC community,

There has been a large, collaborative effort to initiate a cyclodextrin clinical trial at the National Institutes of Health (NIH) to systematically evaluate the safety and efficacy of cyclodextrin therapy for the treatment of Niemann-Pick type C (NPC) disease. As many of you are aware, we met with the Food and Drug Administration (FDA) this past Tuesday, November 1, 2011, to discuss the development program for cyclodextrin

The exceptional work that has been done in NPC animal models has guided the design of a human clinical trial. Together with the Therapeutics for Rare and Neglected Diseases (TRND) group at the NIH, as well as several NPC researchers, Johnson & Johnson, and consultants from RRD International, LLC, we are working to submit an Investigational New Drug (IND) application to FDA.

The first step in submitting the IND application to FDA (the prerequisite to an initial clinical trial in patients) was to request a pre-IND meeting with FDA to receive the Agency's feedback on our development program before the IND application is officially submitted. On November 1 we met with the FDA review division staff to discuss the proposed development plan for cyclodextrin and needs for the IND application package. The meeting was positive and the Agency provided helpful feedback focusing on the drug safety and toxicology data. We will have an additional meeting with FDA to focus on the clinical trial design, and FDA is working with us to get that meeting scheduled before the end of the year.

We view this as a very positive step toward pursuing cyclodextrin as a potential treatment for NPC disease. We are planning a scientifically rigorous trial that will allow us to test cyclodextrin in our patients safely and in a way that will provide as much information as possible. While specific details of the trial will not be available until we have agreement from FDA and approval from the NIH ethics review board, we will share information with the NPC community as it is available.

We continue to work toward our goal of starting the trial next year and feel that with the recent FDA feedback, we are on track to do so.

Thank you for your continued support and encouragement as we work together to find a treatment for NPC disease. This fight would not be possible without all of you.

Sincerely,

The TRND Team