

Good morning,

We are happy to share with the NPD community that the NIH/TRND Cyclodextrin in NPC1 trial has been restarted. Despite the Federal Government shutdown from October 1-16, our team was able to meet our projected re-start date in late September, and to date have coordinated three successful admissions on this protocol. We are grateful for the patience of the participating families, and for the dedication of the NIH Clinical Center staff, which allowed us to avoid any major interruptions in the admission schedule.

We have scheduled three additional patients to start before the end of 2013. The remaining open slots will continue to be scheduled on a rolling basis as we get closer to the start dates in early 2014. We will be re-contacting families who had been screened previously to make sure they are still eligible before we schedule the remaining slots. If any families with children ages 2-25 are interested in more information about the trial or would like a member of our team to screen their child to see if they are eligible, they can contact Nicole (Yanjanin) Farhat at (301) 594-1765 or email nicole.farhat@nih.gov.

Below is a summary of the eligibility criteria for the trial:

Participants must:

- Be between the ages of 2-25 years and have a confirmed diagnosis of NPC1
- Have at least one neurological symptom of NPC1. For example, but not limited to, hearing loss, vertical supranuclear gaze palsy, ataxia, dementia, dystonia, seizures, dysarthria, or dysphagia.
- Be able to travel to the NIH Clinical Center every month for evaluation and follow-up.
- Be on the same dose of miglustat (if taking) for at least 3 months before enrolling in the trial. Dose may not change during the trial.
- Stop all non-prescription supplements (with the exception of an age-appropriate multivitamin) at least 1 month before enrolling in the trial.
- Be willing to participate in all aspects of the trial including lumbar puncture (spinal tap) and blood collections.

Participants are not eligible if they:

- Have received any form of cyclodextrin in an attempt to treat NPC1 before enrollment in the trial.
- Are pregnant or breastfeeding at any time during the study.
- Have a spinal deformity that would impact the ability to perform a lumbar puncture.
- Have thrombocytopenia (a platelet count of less than 75,000 per cubic millimeter).
- Have neutropenia, defined as an absolute neutrophil count (ANC) of less than 1500.
- Have a history/presence of a bleeding disorder.
- Have active pulmonary disease, oxygen requirement or clinically significant history of decreased blood oxygen saturation, pulmonary therapy, or requiring active suction.
- Are unable to complete a behavioral hearing evaluation to monitor for ototoxicity.
- Have difficult to control seizures, specifically if unstable in frequency, type or duration of seizure; those requiring ongoing medication changes to control seizures; or requiring 3 or more antiepileptic medications to control seizures.

NIH/TRND Team
National Institutes of Health