

# NIEMANN-PICK TYPE C1 RESEARCH STUDY

## **New clinical trial for individuals with Niemann-Pick Type C1 (NPC1) and chronic, non-acute liver disease**

The National Institutes of Health is looking for those with NPC1 and chronic, non-acute liver disease to be in a Phase I, open-label research study for an experimental drug called cyclodextrin.

The study will look at the safety of intravenous (IV) cyclodextrin and whether IV cyclodextrin is helpful in treating NPC1-related chronic liver disease.

### **To participate in this study, one must?**

- Be diagnosed with NPC1 and chronic liver disease.
- Be between 4 to 60 years of age.
- Be healthy enough to travel to the NIH every month for 12-18 months
- Be willing to stop taking miglustat (Zavesca) while in the study

### **What is involved?**

- Admission to NIH Clinical Center once a month
- Visits will last 2-5 days
- One will get cyclodextrin through an IV
- Visits will include evaluations, blood and urine collection, imaging and fibroscan
- Participants will be randomly dosed to one of three doses
- One will have the option of receiving intrathecal (IT) cyclodextrin at the NIH during the study.
- There is no cost for study-related tests or procedures
- Lodging and domestic travel compensation will be provided.

## CHRONIC LIVER DISEASE



### **Location:**

National Institutes of Health  
Clinical Center  
9000 Rockville Pike, Bldg. 10  
Bethesda, MD

### **Contact Information**

To find out more about this study, please call toll free:

1-800-411-1222

TTY: 1-866-411-1010

Online,

<https://clinicaltrials.gov/>

NIH study 19-CH-0028