

**Protocol Number:** 19-CH-0028

**Active Accrual, Protocols Recruiting New Patients**

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**Title:** Combined Intrathecal and Intravenous VTS-270 Therapy for Liver and Neurological Disease Associated with Niemann-Pick Disease, Type C1

**Background:**

For people who have Niemann-Pick disease, type C1 (NPC1), cholesterol and other fats have trouble moving out of liver and other tissue cells. This makes the cells sick. Researchers want to find out if a drug called VTS-270 can help.

**Objective:**

To test if VTS-270 is safe and effective in treating chronic liver disease associated with NPC1.

**Eligibility:**

People ages 4–60 with NPC1

**Design:**

Participants may be screened by phone or under another protocol.

Participants will have visits once a month for 12 months. If they have intrathecal injections, the study may last 15 months or more. The first visit will last about 5 days. Others will last 2–3 days. Participants will get VTS-270 injected into a vein at each visit. They can also choose to have intrathecal injections. These are like spinal taps.

Some visits will also include:

- Physical exam

- Urine tests

- Blood tests. A small tube or needle will be inserted into the participants vein to collect blood. The small tube will also be used to give the VTS-270.

- Hearing tests: For one test, participants will have electrodes taped to their head. These will record brain waves.

- Breathing tests

- Ultrasound of abdomen: Sounds waves will take pictures of the participant's body.

- Chest x-ray: This is a picture of the lungs.

**Sponsoring Institution:** Eunice Kennedy Schriver National Institute of Child Health and Human Development