Recruitment Ongoing for Niemann Pick Type C1 Study

CTD-TCNPC-101 - A Phase I study to evaluate the safety, pharmacokinetics and biomarkers of disease with intravenous Trappsol® Cyclo™ (HP-β-CD) administration in patients with Niemann-Pick disease type C (NPC-1).

Study Rationale - Niemann-Pick disease type C (NPC) is a rare, fatal, autosomal recessive lysosomal storage disease characterised by progressive major organ failure and neurodegeneration. It is associated with impaired intracellular lipid trafficking, resulting in the toxic accumulation of unesterified cholesterol and glycosphingolipids in major organs. There are currently no treatments approved for NPC on a global basis. One treatment, miglustat (Zavesca®) is approved for NPC in the EU but not in the US.

CTD Holdings, Inc. is developing Trappsol® Cyclo™ (hydroxypropyl-β-cyclodextrin [HP-β-CD]) for the treatment of NPC. The purpose of this study is to assess the safety of Trappsol® Cyclo™ and effects on markers of cholesterol metabolism and disease when administered at doses of up to 2500 mg/kg by slow IV infusion over 8-9 hours every two weeks.

Objectives include:

To compare the plasma and cerebrospinal fluid (CSF) pharmacokinetics of 2 different doses of Trappsol® Cyclo™ in patients with NPC-1 after single and multiple infusions

To investigate the effect of 2 different doses of intravenous Trappsol® Cyclo™ in patients with NPC-s disease upon serum and lymphocytic markers of cholesterol metabolism

To evaluate treatment-related adverse events

To evaluate the effect of 2 different doses of Trappsol upon change in clinical manifestations of NPC-1 disease.

Patients and Methods:

- Phase I
- 12 patients will be recruited
- Patients will receive treatment every 2 weeks for a 14 week treatment period. Eligible patients may continue receiving the drug with the approval of the physician and the sponsor.

Key Inclusion	Key Exclusion
- Confirmed diagnosis of NPC Type C	- Receipt of cyclodextrin therapy within 3 mo. of baseline
- Aged 18 years upwards	- Concurrent treatment with therapy that lowers cholesterol
- NIH NPC Severity Score <30	- Stage 3 chronic kidney disease (CKD
	- Evidence of acute liver disease

Recruiting Sites:

UCSF Benioff Children's Hospital Oakland, CA
Contact: Dr. Caroline Hastings
Email: chastings@mail.cho.org

Morristown Medical Center, Morristown, NJ
Contact: Dr. Darius Adams
Email: Darius.Adams@atlantichealth.org

Physicians may also contact:
Sharon Hrynkow PhD, Chief Scientific Officer, CTD Holdings
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Patients and Families may also contact:
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