Update on the clinical trial of VTS-270: The NPC Study

Please join us for a webinar as Dr. Forbes Porter of the NIH shares the 18 month data from the Phase 1/2 evaluation of intrathecal 2-hydroxypropyl-β-cyclodextrin for the treatment of Niemann-Pick disease, type C1

**When:** Friday, May 20th 12:30 – 1:00pm ET  
**Where:** Webinar – please email carrie@vtessepharma.com to register. If you cannot make the live webinar please indicate that in your email and we will send you the recorded webinar.

**WHY VTS-270**

The NPC Study is a Phase 2b/3 randomized, sham-controlled clinical trial designed to evaluate the efficacy and safety of VTS-270 (a proprietary form of cyclodextrin) for the treatment of children with Niemann-Pick Disease Type C (NPC). Its primary objective is to evaluate the progression of the neurologic manifestations of NPC in children treated with VTS-270 compared to those who do not receive the drug.

**VTS-270, THE ONLY INVESTIGATIONAL DRUG**

- **TO DEMONSTRATE STRONG SURVIVAL BENEFIT IN MULTIPLE ANIMAL MODELS**
  - ... when treatment is started early  
  - ... when treatment is started after the progression of symptoms

- **TO DEMONSTRATE PRESERVATION OF NEURONS**
  - ... in naturally occurring animal models of the disease

- **TO HAVE BEEN CLINICALLY TESTED IN NPC PATIENTS**
  - Demonstrating emerging positive effects on clinically-relevant function, based on serial analyses from the Phase 1/2 trial and compassionate use/IRD studies

- **TO BE SAFE AND WELL-TOLERATED IN STUDIES TO DATE**
  - In NPC patients, high frequency hearing loss reported as a side effect with minimal and manageable impact on quality of life that can be corrected with hearing aids

- **TO HAVE BEEN GRANTED BREAKTHROUGH STATUS BY FDA**
  - With a group pivotal trial that is currently enrolling patients. The trial is currently enrolling patients.  
  - (Up to 20 sites worldwide)

**AND...**

- **12 MONTHS**
  - Study design ensures that the controlled portion of the study is limited to 12 months for any single subject

- **COMPREHENSIVE BATTERY OF PRECLINICAL STUDIES**
  - Have not only demonstrated safety and positive effects but also predicted dosing levels, frequency and route of administration for the pivotal clinical trial

- **CLINICAL TRIAL DOSES**
  - VTS-270 will be tested in ranges that are predicted to be safe and efficacious from the animal model studies and Phase 1 clinical trial

- **INTRatheCAL ADMINISTRATION**
  - Essential for passive effects in the brain with strong dose-dependent responses seen for this route of administration (device under development)

www.thenpcstudy.com