



## 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- $\beta$ -cyclodextrin for the treatment of Niemann-Pick disease, type C1

**When:** Friday, May 20<sup>th</sup> 12:30 – 1:00pm ET

**Where:** Webinar – please email [carrie@vtessepharma.com](mailto: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 initial analyses from the Phase 1 trial and from compassionate use/IND studies



#### TO BE SAFE AND WELL-TOLERATED IN STUDIES THUS FAR...

... 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 to date with hearing aids



#### TO HAVE BEEN GRANTED BREAKTHROUGH STATUS BY FDA...

...with a global pivotal trial that is currently treating patients. **THE TRIAL IS CURRENTLY DOSING PATIENTS.** (Up to 20 sites worldwide)

### AND...



#### STUDY DESIGN ENSURES

that the controlled portion of the study is limited to 12 months for any single subject



#### COMPREHENSIVE BATTERY OF PRECLINICAL STUDIES

but also predicted dose 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 positive effects in the brain with strong dose dependent response seen for this route of administration (device under development)