Vtesse

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

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/iIND 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
have not only demonstrated safety and positive effects 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)

www.thenpcstudy.com

Update on the Vtesse Clinical Trial of VTS-270:

Vtesse is pleased to announce that Part A is fully enrolled. Part A studied three different doses, 900mg, 1200mg, and 1800mg, and is designed to identify the dose selected for Part B. This decision is currently scheduled to take place in the May 2016 time frame, after which, patients will be enrolled into Part B.

In the meantime, screening for inclusion into Part B of the study is currently ongoing. Vtesse has opened two sites in the UK, one site in Spain, six sites in the United States, and progress is continuing on opening sites in Germany, France, Turkey, Australia, and also additional US sites.

Patients who complete Part B of the study will be rolled over into Part C of the study that is open-label. That is, in Part C, all patients in the trial will receive VTS-270 as we await regulatory decisions.

To find out more about the trial and to find a clinical trial site please visit www.theNPCstudy.com or https://clinicaltrials.gov/ct2/show/NCT02534844?term=vtesse&rank=1.

"VTS-270 Clinical Trial Patient Experience" video:

Many people have been asking, "what is it like to be in the trial?" In response to that question, Vtesse has launched an educational video that seeks to help families better understand what it is really like to participate in the clinical trial of VTS-270. The video is currently being translated into German, French, Spanish (Latin American), Spanish, Turkish, and Italian. The video can be viewed at www.theNPCstudy.com or at https://vimeo.com/160183927.

18 month data available from the NIH Phase 1 clinical trial:

The data were presented by Dr. Forbes Porter at the 2016 World Symposium on Lysosomal Storage Disease in San Diego, California. Dr. Porter will also present the data at the APMRF meeting in June 2016.

The Phase 1/2 clinical data from 14 patients with Niemann-Pick Type C1 showed that after 12 months and 18 months of treatment with VTS-270, disease progression as measured by the NPC Clinical Severity Score (or NPC-CSS, which looks at, among others, ambulation, fine motor ability, cognition, speech, memory and swallowing) was significantly reduced compared to data from a control group who did not receive VTS-270.

"We are encouraged by this preliminary Phase 1/2 data, showing a clear effect on severity of symptoms from Niemann-Pick Type C1 disease," said Ben Machielse, Drs., President and Chief Executive Officer of Vtesse, Inc. "We believe this shows that VTS-270 has the potential to substantially slow down disease progression for NPC1, which supports our continuing clinical study in the hope that we can apply for regulatory approval of VTS-270 as a treatment for the patients in need."

Update on the device for administering VTS-270:

Many of you have asked for a delivery device for VTS-270. Vtesse has formally begun working on getting a device ready to be introduced during Part C of the trial in Europe. The device is already approved by EMA but has not been approved yet by FDA. The goal is to get the device approved by FDA and have it introduced in the United States as well. We will also work to get approval in other countries such as Turkey, Australia, and Canada.

The device is an intrathecal portal (or port). It is an implanted device that allows access to the intrathecal space. These are used regularly for delivery of medications for pain management. A device would eliminate the need for lumbar punctures.