



Update on the clinical trial of VTS-270: The NPC Study *July 2016 Newsletter*

Update on the Vtesse Clinical Trial of VTS-270:

There continues to be positive momentum in enrollment, but we still need additional patients to participate in the trial. Of note, two families from China have relocated to the United States for the clinical trial and one family from India is in the process of relocating to the United States for the trial. The patients from China have been enrolled. This is quite significant and shows the level of motivation some have for enrolling in this trial.

Clinical Trial Site Expansion Update:

Vtesse also successfully expanded its clinical trial into Turkey. Two sites in Turkey have begun screening patients for the trial. In addition, Dr. Del Toro in Spain has enrolled the first patient at her site and will be recruiting additional patients for the trial. We also continue to work on opening additional sites in the United States and Germany. Vtesse also anticipates expansion into Australia.

NNPDF Industry Survey:

Late 2015, NNPDF contacted Vtesse and asked us to fill out a questionnaire about the clinical trial of VTS-270. They have recently posted the results of the survey. The information below is taken from the NNPDF website and explains the survey. In addition, a link to the survey is included below.

“In September of 2015, the following questionnaire was sent to CTD Holdings, Orphazyme and Vtesse by the National Niemann-Pick Disease Foundation (NNPDF) for their review, completion and comments. The intent was to:

a) Allow for objective side by side comparison of the active clinical trials based on information

provided by the companies from a pre-clinical, clinical and scientific perspective.

b) collect the science behind the drug along with the trial details so that this information can be provided to family doctors and consultants who are treating patients with Niemann Pick Type C so that they can help guide patients who are interested in participating in a trial, and

c) to ensure that any pharmaceutical company wanting to run a trial on our children and young adults have done their due diligence in ensuring the safety of the drug and have done everything they can to ensure a favorable outcome.

The foundation wishes to ensure that companies provide the Niemann-Pick Disease community with as much information as possible so that all, parents, treating physicians and medical teams can make informed decisions. This survey form is intended to be utilized in the future of any company that plans a drug trial in the future. This is our expectation.

The list of questions was compiled from several sources including the National Cancer Institute and the NIH. Additional questions were added tailored to NPC. The questionnaire is broken down into 4 parts: Part 1: Clinical Trial Questionnaire for Niemann-Pick Disease Type C; Part 2: Treatment Rationale / Pre-clinical Studies; Part 3: Safety/Dosing and Part 4: Clinical Trial / Protocol

The document has a lot of details about the science of the drug which sometimes is not easily understood. For these types of responses, the answers in the table were condensed and a link is provided which you can click on to get additional information regarding the questions.

It is the intent of the NNPDF to have any company that plans a drug trial in the future to fill out this questionnaire.”

You can find the industry survey at:

http://www.nnpdf.org/documents/ClinicalTrialSurvey06032016revisedFINAL_002.pdf

Vtesse Webinars:

Vtesse conducted a Town Hall Webinar focused on the blood brain barrier and an update on the Phase 1 data. The recording will be available for viewing shortly. If you were not able to attend the webinar in person and would like to view it, it will be sent out through the advocacy groups or you can contact us directly at carrie@vtessepharma.com. Also, if there are topics you would like discussed during future webinars, please let us know via the email above or send those your local NP Family Organization. We want to ensure these webinars include information that is valuable to you, so please send us your input.

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>.

