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September 28, 2015

RE: **Vtesse, Inc. Initiates Phase 2b/3 Clinical Trial of VTS-270 for Treatment of Niemann-Pick Type C1 (NPC) Disease**

Dear NNPDF NPC Patient and Family Community,

The National Niemann-Pick Disease Foundation (NNPDF) is pleased to advise our Niemann-Pick Disease Type C patient & family community that Vtesse, has received FDA authorization to begin recruiting for the Cyclodextrin / VTS-270 clinical trial for patients in the United States, diagnosed with NPC1, who fall within the age range of 6 to 21 years.

The first Cyclodextrin / VTS-270 clinical trial site for the US has been established at Rush University Medical Center in Chicago, Illinois under the direction of Elizabeth Berry-Kravis MD, Ph.D. and the screening of the first three patients for inclusion in the trial has begun. We have included a copy of the full press release announcement from Vtesse below.

The official name of this trial is: **"Study of 2-hydroxypropyl-β-cyclodextrin (VTS-270) to Treat Niemann-Pick Type C1 (NPC1) Disease"** and has been assigned the clinical trial number of: **NCT02534844** on ClinicalTrials.gov. To learn more about this trial, please consult with your physician and refer to the web site link: https://clinicaltrials.gov/

This therapy is a Phase 2b/3 trial which is a multi-center, multi-national, trial for patients with NPC1 under one global protocol that will evaluate safety and efficacy of VTS-270 to support approval of the drug by regulatory agencies in both the United States and Europe. A recap and history of the Cyclodextrin / VTS-270 Phase 1 clinical trial can be found at the NNPDF web site via the following link: http://nnpdf.org/Vtesse.html

As we look to the future, Vtesse will be rolling out more clinical trial sites around the country. As soon as this information is made available to the NNPDF, we will be certain to share this information with our NPC patient and family membership community.

As always, should you have any questions regarding this announcement, please feel free to contact the NNPDF Central Office.

We WILL Persevere in our Quest for a Cure!

Sincerely,

Nadine M. Hill
Executive Director
National Niemann-Pick Disease Foundation
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NOTE: None of the above signatories engage in the practice of medicine, nor do we claim to be a medical authority or claim to have medical knowledge. This document is designed to be an educational service and is not meant to provide diagnostic or treatment advice. Information contained or suggested on this document does not constitute medical advice. For all information related to care, medication or treatment, it is recommended that you consult a physician to determine if the information presented is applicable.

It should also be noted that choosing to participate in a clinical study is an important personal decision. Talk with your doctor and family members or friends about deciding to join a study. To learn more about this study, you or your doctor may contact the study research staff indicated on Clinicaltrials.gov once the trial begins to recruit patients. For general information about taking part in a clinical trial please see: The US Government Web Page Titled: Learn About Clinical Studies via this link: https://www.clinicaltrials.gov/ct2/about-studies/learn

FOR IMMEDIATE RELEASE

Vtesse, Inc. Initiates Phase 2b/3 Clinical Trial of VTS-270 for Treatment of Niemann-Pick Type C1 (NPC) Disease

- *During its first year of operations, Vtesse advances lead program into late-stage, pivotal trial and launches www.theNPCstudy.com*
- *Goal is to establish a clear foundational data set on the safety and efficacy of VTS-270 for treatment of NPC*

Gaithersburg, MD, September 28, 2015 – Vtesse, Inc. today announced that the first three patients have been screened for inclusion in its pivotal Phase 2b/3 clinical trial with VTS-270 for treatment of Niemann-Pick Type C1 Disease (NPC). This clinical trial follows a Phase 1 study conducted by researchers at the National Institutes of Health (NIH) Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD).

NPC is a progressive, irreversible, chronically debilitating – and ultimately lethal – genetic disease. It is caused by a defect in lipid transportation within the cell, which leads to excessive accumulation of lipids in the brain, liver and spleen. Vtesse has worked extensively with regulators in the United States and Europe with the goal of conducting its pivotal study under one global protocol that will evaluate safety and efficacy of VTS-270 to support approval of the drug by regulatory agencies in both regions.

Ben Machielse, Drs., President and Chief Executive Officer of Vtesse, Inc., said, "Starting the pivotal clinical trial for VTS-270 within nine months of the company's launch underscores our dedication to serve the NPC community. A key ingredient that enabled us to start the trial so quickly is the complete package of high-quality pre-clinical data that clearly outlines the potential benefits of VTS-270, such as prolonged survival and preservation of neurons, as well as encouraging clinical data from the Phase 1 trial. The pivotal trial will allow us to further evaluate safety and efficacy of VTS-270 in the pivotal trial to build a foundational dataset for the NPC community."

He added, “While initiation of the first site at Rush University Medical Center and the screening of three patients is a key milestone, we intend to open several sites in quick succession in the United States and the European Union to ensure availability of sites in close proximity to the patients who are eligible to enroll in the trial.”

Vtesse’s Phase 2b/3 prospective, randomized, double-blind, sham-controlled trial of VTS-270 will be conducted in patients affected by NPC disease. The trial will take place in up to 20 centers in the United States and Europe. It is a three-part, efficacy and safety trial of VTS-270, administered by the lumbar intrathecal route (IT) every two weeks, with a planned enrollment of approximately 51 patients (in Parts A and B). In Part A of the study, researchers will evaluate three different dose levels of VTS-270 versus sham-control to determine the dose level for Parts B and C. All participants in the pivotal trial will be eligible to receive treatment with VTS-270 in Part C, the open-label extension, until the time of regulatory decisions.

Preliminary analyses of the Phase 1 trial, conducted post-hoc, suggest that the rate of disease progression had slowed down (based on a standardized measure) in children treated with VTS-270 as compared to the rate in an age- and disease severity-matched cohort obtained from a separate natural history study of NPC patients.

The VTS-270 pivotal study addresses a pressing need to discover new treatments for the disease, according to Forbes D. Porter, M.D., Ph.D., Co-Principal Investigator of the Phase 2b/3 study, Senior Investigator and Program Head in the intramural research program of the Developmental Endocrinology and Genetics Program (PDEGEN), NICHD. He added that the trial has the potential to provide important information about the disease as well as how patients may tolerate VTS-270 and its potential effects on the symptoms of NPC.

“The recent analysis of the preliminary Phase 1 clinical data for VTS-270 has provided encouraging support suggesting this treatment for NPC may be able to alter the course of this fatal disease,” said Elizabeth Berry-Kravis, M.D., Ph.D., Co-Principal Investigator

of the Phase 2b/3 study, and Professor of Pediatrics, Neurological Sciences, Biochemistry, Rush University Medical Center. “The beginning of patient screening for the pivotal Phase 2b/3 study for VTS-270 marks another important step in the journey toward targeted treatment of NPC, as clinical trials are the only means to determine the effectiveness of VTS-270. This pivotal trial will allow us to gather the data required to advance the regulatory process and move toward U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) approval for VTS-270."

More information on this Phase 2b/3 study can be found at Vtesse’s clinical trial website: www.theNPCstudy.com. Vtesse launched the website this summer as a resource to patients, parents, caregivers and physicians. For more information on enrollment criteria, please see: www.theNPCstudy.com/ - !trial-enrollment/c1ine.

About VTS-270

Vtesse’s lead compound, VTS-270, has shown promise in pre-clinical and clinical studies as a potential treatment for NPC. It is a well-characterized mixture of (2-hydroxypropyl)-beta-cyclodextrin that has been extensively evaluated in pre-clinical and clinical studies at the National Institutes of Health, as well as under individual compassionate-use Investigational New Drug applications (iINDs) and in other academic labs. Most recently, Vtesse announced positive clinical results from the on-going Phase 1 study that demonstrated VTS-270 stabilizes the overall measures of NPC while improving certain disease measures. NPC is a genetic disease affecting an estimated one in 100,000 to 150,000 children and is often misdiagnosed and/or under-diagnosed. Affected patients are usually identified in early childhood with ataxia, exhibit progressive impairment of motor and intellectual function, and often die before adulthood.

About Vtesse

Vtesse, Inc. is a rare disease company dedicated to developing drugs for patients suffering from diseases that are underserved. Vtesse is working collaboratively with the NIH and other leading academic centers to advance clinical study of VTS-270 for NPC,

and to conduct pre-clinical discovery and development of other novel drugs for NPC and other lysosomal storage diseases (LSDs). Vtesse is led by a highly experienced management team that has been involved in the development of more than 20 approved drugs and vaccines. Its experienced consortium of investors, led by New Enterprise Associates, has committed initial funding that is expected to bring this compound through pivotal clinical trial. Vtesse is based in Gaithersburg, Maryland and is the first spin-out company from Cydan Development, Inc. For more information, visit www.vtessepharma.com.

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