National Niemann-Pick Disease Foundation, Inc.
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Dear NNPDF NPC Community,

August 17th, 2015

RE: Vtesse, Inc VTS-270 (2-hydroxypropyl-beta-cyclocextrin) clinical trial

As the National Niemann-Pick Disease Foundation (NNPDF) Niemann-Pick Disease Type C (NPC) Patient Community looks forward to advances in research and clinical therapies for our loved ones diagnosed with NPC, the various NPC research and patient advocacy directed agencies recently gathered together to identify common ground and clarify specific efforts which will best serve the NPC community. As such, an NPC parent/patient community alignment team has been developed to assist towards efforts in raising awareness and educating the NPC community about upcoming trials and research advances.

The attached letter, signed by the National Niemann-Pick Disease Foundation, Ara Parseghian Medical Research Foundation, Hide & Seek Foundation and Dana's Angels Research Trust outlines the role that we see this NPC community parent/patient alignment team taking on and we ask that you, as an important part of the NPC family community, review this document and add your support to this important effort on behalf of your loved one living with NPD, as well as, the entire NPC family.

Vtesse has worked diligently to coordinate with the FDA (United Stated Food and Drug Administration) and the EMA (European Medicines Agency) to match the trial protocols for the upcoming VTS-270 (2-hydroxypropyl-beta-cyclocextrin) clinical trial. The FDA/EMA trial protocols mandate a minimum of **51 patient** enrollees to fulfill the trial recruitment goal ~ thus the team project name of: **"51 & Done!"** In addition to adding your support to this effort through your signature, we also invite you to join us in this important awareness campaign through other activities! First up, a lapel button with the **"51 and Done!"** logo (see design below) which has been developed and are available at the NNPDF Central Offices. Simply e-mail the desired quantity and mailing address to: nnpdf@nnpdf.org and we will forward along to your home.

We have also enclosed a press release from Vtesse, Inc., dated August 6th, 2015 which further details clinical patient findings from the ongoing NIH Phase 1 Study of VTS-270 (2-hydroxypropyl-beta-cyclodextrin).

In closing I just ask you to remember a quote form Helen Keller "Alone We Can Do So Little, Together We Can Do So Much". **We WILL Persevere in our Quest for a Cure!**

Sincerely,

Nadine M. Hill
Executive Director
National Niemann-Pick Disease Foundation
nhill@nnpdf.org

August 4, 2015

Dear NPC Patient Community:

It is hard to imagine how far we have progressed with the research and development of potential treatments for Niemann-Pick C. The entire community deserves to be commended on the hard work and dedication it has taken in order to get us to this point. Our community is in a position that many other rare disease communities strive to achieve. Researchers, companies, and even investors are focusing on developing treatments for NPC.

This influx of activity leaves us in a position of great hope, but at the same time, it also leaves us in a position of great concern. The concern is this: can this small patient community support potentially three clinical trials all at the same time? It is one of those "good problems to have," but if the answer is "no," then our hopes and our chances of having approved products for NPC patients are greatly diminished.

Clinical trials need to be able to fully enroll patients and follow those patients throughout the study in order to gather the data needed for potential approval. Given the small number of potentially eligible patients for these studies, and, as leaders in the NPC community, we are concerned about the real possibility that our patient community won't be able to recruit the number of patients necessary to fully enroll the continuation of the NIH's Phase I Cyclodextrin trial. As such, we encourage the NPC community to support the upcoming Vtesse clinical trial of VTS-270 (Cyclodextrin) as a stand-alone therapy for NPC patients who qualify within the pre-determined inclusion criteria.

Vtesse has worked very hard to get a protocol agreed upon by the FDA (United States Food and Drug Administration) and EMA (European Medicines Agency). This is important because if the protocols are different for the United States vs Europe, then twice the number of patients are needed to complete the studies. For example, if one of the agencies didn't agree upon a protocol, then instead of needing 51 patients to complete the study, we would need 102. That would further diminish the pool of patients for any other trial. Enrollment and completion of the trials would take longer and ultimately patients who are not eligible for the trial would have to wait even longer for the chance to have an approved product. Also, having a protocol, which is approved in the USA and Europe, will prevent a situation where a drug will be developed that is only available (and potentially reimbursable) in one of these territories. It is critical that a drug is developed with the potential for the broadest access possible.

Several other factors went into making this decision. First, not all hydroxyl-propyl-betacyclodextrins are the same. The cyclodextrin used in the NIH Trials is the same cyclodextrin formulation the Vtesse will use in their trial, VTS-270 which was evaluated in extensive pre-clinical studies and makes this the only well characterized formulation of consistent quality.

Second, as a result of the Vtesse trial, cyclodextrin would serve as a reference drug, thus eliminating the need for a no drug control group in future studies. This could speed up future drug development, with cyclodextrin being the first and essential part of a combination, "drug cocktail" treatment for NPC.

Third, if we are unable to take advantage of it and achieve full enrollment for this trial, it will tar our reputation and serve as a disincentive for future drug development and clinical trials.

The NPC community is extremely fortunate to have this opportunity. If the NPC patient community can achieve full enrollment for this trial, it will enhance the community's opportunities and reputation within the rare disease community, which will bode well for the entire NPC community in the future, including prospective drug development and clinical trials.

Fourth, Vtesse has shown a commitment to the NPC patient community. They have kept the lines of communication open and have listened and incorporated patient feedback into their planning. For instance, Vtesse learned through community feedback the need to ensure that patients currently being prescribed miglustat be evaluated for inclusion in the trial and be able to continue on with this medication. They have also incorporated a rescue option for patients included in the trial but randomized to the control arm of the trial. More specifically, should the condition of these randomized patients deteriorate while in the trial, they would be channeled to receive access to the medication. Vtesse is also investigating the development and incorporation of an alternative and less invasive delivery application and device, and looking into how to optimize VTS-270 to improve the safety and efficacy profile.

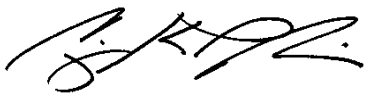
Last, but not least, Vtesse has brought community and scientific leaders together in order to stimulate dialogue on how our community can be successful in drug development for NPC. It is clear by their actions that their commitment to our community is real. This trial is one step towards getting our patients the treatments they need in order to most effectively manage and one day cure NPC.

If Vtesse can establish a way forward for global drug development for NPC, this could open the door for others to expand the treatment options for NPC patients. The last thing we want is to be this close to gathering the data needed to assess the safety and efficacy of cyclodextrin for NPC, and yet, not be able to gather the data due to insufficient participation in the clinical trial.

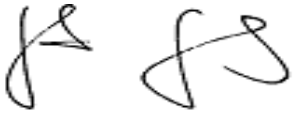
In closing, and based on the foregoing, we the undersigned, support the Vtesse clinical trial to assess the safety and efficacy of VTS-270 in NPC, and hope that you will do the same.

We therefore ask you – and your family's foundation if possible – to join us in signing this letter as a sign of our united commitment to bringing this ground-breaking therapy to everyone with the horrible disease that has afflicted our children.

Best wishes,



Cindy K. Parseghian
President
Ara Parseghian Medical Research Foundation



Jonathan Jacoby
Chair
Hide & Seek Foundation

Phil Marella
Trustee
Dana's Angels Research Trust (DART)

Leslie Hughes
Board Chair
National Niemann-Pick Disease Foundation

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

Vtesse, Inc. Announces Preliminary Data from Ongoing Phase 1 Study of VTS-270 for Treatment of Niemann-Pick Disease Type C

Post-Hoc Analyses of Data Show Preliminary Evidence of Overall Disease Stabilization with Improvement in Several Disease Domains

Gaithersburg, MD, August 6, 2015 – Vtesse, Inc. announced preliminary results today from an open-label Phase 1 clinical trial with VTS-270 (a formulation of (2-hydroxypropyl)-beta-cyclodextrin) for treatment of Niemann-Pick Disease Type C (NPC) conducted by researchers at the National Institutes of Health (NIH) Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD). Preliminary analyses, conducted post-hoc, suggest that the rate of disease progression had slowed down (based on a standardized measure) in children treated with VTS-270 in the Phase 1 trial as compared to the rate in an age- and disease severity-matched cohort obtained from a separate natural history study of NPC patients. The analyses also show that children treated with VTS-270 demonstrated improvement on several disease domains.

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. Researchers at NIH's National Center for Advancing Translational Sciences (NCATS) and NICHD, in close collaboration with Vtesse, patients and patient advocacy groups, developed VTS-270 as part of a project focused on finding treatments for NPC. VTS-270 has been shown to significantly reduce disease progression in naturally occurring animal models and is currently being tested in this Phase 1 clinical trial. Both the ongoing Phase 1 study and the natural history study use a standardized measurement of disease progression, the NPC score, which relies on a specific rating scale that scores the disease along major domains (or traits).

Researchers have matched participants in the Phase 1 study to individuals from the natural history data set according to baseline age and disease severity. The rate of disease progression of this matched cohort was compared to the rate of disease progression of children treated with VTS-270 in the Phase 1 study.

In the Phase 1 trial, among the 12 children treated with direct administration of VTS-270 into the cerebrospinal fluid via an intrathecal administration for more than six months and up to 12 months, the overall NPC score showed a slowing down in the rate of decline. In these patients, when the scores for impact on hearing were removed, the NPC score showed disease stabilization and halting of progression. Domains of cognition and speech have shown improvement of the disease state for participants in the study; while ambulation, fine motor skills, cognition, swallowing, and memory demonstrate slowing down of decline. Measurements of eye movement and hearing appeared to have worsened among participants in the Phase 1 trial.

"This initial analysis of the data look encouraging and this therapy may make a meaningful difference for children with NPC," said Ben Machielse, Drs., President and Chief Executive Officer of Vtesse, Inc. "Based on discussions with regulators, we anticipate that a single pivotal trial would form the basis for approval of VTS-270 in the United States and Europe. The pivotal trial design will be randomized and controlled, and our planned implementation of the trial underscores the essence of our commitment to the NPC patients and their

caregivers. We intend to execute flawlessly and with the greatest care for this sensitive group of patients who are in dire need of new treatment options."

In the Phase 1 trial to date, VTS-270 has been well tolerated other than observed worsening in the eye movement and hearing of some trial participants. Clinicians have administered more than 250 intrathecal administrations of the drug to study participants thus far with minimal administration-related side effects. Vtesse plans to submit more complete Phase 1 clinical trial results for presentation at a scientific meeting later this year. More details about the study can be found athttps://www.clinicaltrials.gov/ct2/show/NCT01747135.

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 NIH, as well as under individual compassionate use investigational new drug applications (iINDs) and in other academic labs. 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. Vtesse is working expediently with NIH's National Center for Advancing Translational Sciences (NCATS) and NICHD, regulatory authorities, patient/parent organizations, physicians and other key stakeholders to soon start a Phase II/III clinical trial to assess the efficacy of the compound for the treatment of NPC. Based upon productive discussions with U.S. Food & Drug Administration (FDA) and the European Medicines Agency (EMA), Vtesse will anticipates that this trial will begin enrolling patients in September 2015.

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 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 trials. 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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