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.

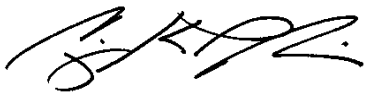
Last, but not least, Vtesse has brought community and scientific leaders together in order to stimulate dialogue on how our community can be successful in drug development for NPC. It is clear by their actions that their commitment to our community is real. This trial is one step towards getting our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



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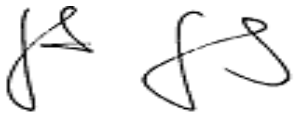

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NOTE: None of the above signatories engage in the practice of medicine, nor do we claim to be a medical authority or claim to have medical knowledge. This document is designed to be an educational service and is not meant to provide diagnostic or treatment 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