Dear NPC Community,

As Global Principal Investigator and Head of the Global Steering Committee for the Phase 3 study TransportNPC™, it is my pleasure to update you on our progress to date to advance this pivotal study. TransportNPC™ is a double-blind, randomized, placebo-controlled, parallel-group, multicenter study to evaluate the safety, tolerability, and efficacy of Trappsol®Cyclo™ (hydroxypropyl-beta-cyclodextrin) and standard of care compared to placebo and standard of care in patients with Niemann Pick Type C1. This is the most advanced program underway for NPC, and it follows directly from our successful Phase 1 and 2 programs.

- So far in 2023, we have expanded this study to include several additional countries including UK, Italy, Brazil, Argentina, and Taiwan meaning the study is now active in 12 countries around the world.

- We have activated more clinical trial sites in the United States, and plan to add two more sites this year.

- In the U.S., participants may be able to receive some of their treatments at home, helping to alleviate some of burden we know comes with participating in a clinical trial. We worked with our clinical trial sites in the US to add this option as a direct response from families expressing the difficulty of travel on patients and caregivers.

- Enrollment is steady and half-way completed. We expect to complete enrollment by the end of 2023.

- Patients are progressing through scheduled visits, and some will have more than one year of data by the end of this year. These data are crucial to understanding how hydroxypropyl-beta-cyclodextrin may work within the body.

Together with you, we have steadily increased our understanding of NPC and Trappsol®Cyclo™. We first provided this investigational therapy to address symptoms of NPC back in 2009. This was a compassionate use program begun in the US which expanded in 2010 to several other countries. Our formal clinical study began in 2015. Today, I am joined by physicians from 11 other nations to advance this program as quickly as possible in our collective global effort aimed at bringing a much-needed approved treatment to the NPC community.

To the heroic study participants and their devoted families, we know it is not easy to commit to a clinical study especially given the day-to-day challenges that come with NPC. I am pleased at the progress we have made together to advance this study. Thank you for sharing your journey with NPC with us, we are devoted to making your time and commitment count.

With sincere appreciation,

Caroline Hastings, MD
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Director, Pediatric Hematology Oncology Fellowship Program
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