



Dear Members of the NPC Community,

My name is Scott Fine and I have been CEO of Cyclo Therapeutics since 2015. Our company is enrolling a Phase 3 study of a potential treatment for NPC. The study, called "<u>TransportNPC</u>", is the most advanced program underway for NPC, and it follows directly from our successful Phase 1 and 2 programs.

The needs that exist for those living with NPC, and their loved ones, are immeasurable. As a father of four, grandfather of six, and now as a caregiver to my wife of 44 years, I lead my life with empathy. Not a day goes by when I don't think about the burden of this disease on the NPC community. As a company and as a team of professionals who are passionate about helping the NPC community, we have made a commitment to work tirelessly to advance a potential treatment option for this devastating disease.

This commitment is not new, and our progress is tangible:

- 2009: We provided the first patients with investigational hydroxypropyl-beta-cyclodextrin as a potential therapy to address the symptoms of NPC. This was a compassionate use program begun in the US which expanded in 2010 to several other countries.
- 2015: We began our formal clinical program to study hydroxypropyl-beta-cyclodextrin, working with the early leaders in the field, especially Dr. Caroline Hastings and Dr. Benny Liu, for our US program. We were joined by leaders in Europe and Israel as we expanded our trials to those countries.
- Today: More US physicians, including Dr. Loren Pena at Cincinnati Children's and Dr. Ozlem Goker-Alpan at Lysosomal and Rare Disorders Research and Treatment Center in Virginia and Dr. Damara Ortiz at UPMC Children's Hospital in Pittsburgh, have joined physician-scientist counterparts from Australia, Israel, Germany, Italy, and other nations to conduct our global pivotal trial, the last trial prior to our hopeful market registration. Dr. Hastings has been with us at every step and is today the global principal investigator and head of the global steering committee for the pivotal trial, a real testament to her dedication and belief in our investigational therapy.

In addition to having to manage NPC, you have endured additional hardships as you await therapies to be approved in the US and around the world. I admire that you have not stood passively on the sidelines. And neither have we. We have been a steady partner, and have listened to the community at every step. You have my commitment that Cyclo will continue to progress our trial with the view toward bringing our drug over the finish line. Should you have questions about our program and progress, or about the individuals who lead our teams, we will respond quickly and transparently.

Our head of patient advocacy is Lori McKenna Gorski and she can be reached at <u>lori.gorski@cyclodex.com</u>. If you 'd like to speak with me directly, please let Lori know and she will put us in touch.

We are privileged and humbled to be on this journey with you, and we look forward to pressing forward with our clinical program for the benefit of NPC community.

Respectfully, Scott

6714 NW 16th Street, Suite B Gainesville, FL 32653

info@cyclotherapeutics.com cyclotherapeutics.com p: 386.418.8060 f: 321.244.8351