



## Hello from Cyclo Therapeutics!

Hello and Happy Spring! We hope that you and your loved ones are well. We are continuing to make great progress with our Phase 3 study for NPC known as TransportNPC™. We are 40 percent enrolled and still expect to complete enrollment by the end of the year. We know that life with NPC can bring challenges, and your engagement with us means everything—thank you. In this newsletter, we are sharing some services and resources that are available to the US NPC Community. Do you have other resources that have helped you in your journey? Advice for others? Let us know if there are other programs or ideas that we should share with the community. We'd love to hear from you.

*-Lori and The Cyclo Team*

Lori McKenna Gorski  
Global Head of Patient Advocacy



## Home Infusion Available in TransportNPC™ Study for US Sites



Joseph Mejia, MD  
Senior Medical Science Liaison, US & LATAM

We are continuing our Phase 3 study, TransportNPC™ (Intravenous 2-hydroxypropyl-β-cyclodextrin), to evaluate the safety and efficacy of a potential disease modifying therapy to treat NPC. We understand that participating in a clinical trial is a big commitment, both for patients and their caregivers. We are pleased to share that within the United States, participants in this study now have the option of receiving their biweekly infusions at home. Here, Dr. Joseph Mejia, Cyclo's Senior Medical Science Liaison, discusses what this new option provides.

### *What does the home infusion option mean for patients?*

For participants in the TransportNPC™ study, while some visits to your clinical trial site are still required, many of your infusions may be given at home. We hope giving patients and caregivers the option to remain at home will significantly reduce travel and daily living disruptions and allow patients to receive the study infusion in the comfort of their home surrounded by loved ones and within a familiar environment.

### *Why was this added as a part of the TransportNPC study?*

We understand the commitment required for patients and families to participate in a clinical trial, and living with NPC already brings daily challenges, appointments to juggle, etc. We worked with our clinical trial sites in the US to add this option because we have heard from caregivers that this could help them, and we want to be sure patients and families remain at the heart of the decision-making process as they voluntarily participate in our clinical trial.

### *How can I find out more?*

If you would like more information about TransportNPC™'s home nursing model, the best place to start is with speaking with your treating physician or by contacting a TransportNPC™ clinical trial location near you. Finally, you can always reach out to Lori, who oversees Patient Advocacy at Cyclo Therapeutics at [lori.gorski@cylodex.com](mailto:lori.gorski@cylodex.com) with any questions!



## Family Services and Support at NNPDF: Meet Laurie Turner

The National Nieman-Pick Disease Foundation (NNPDF) is a patient advocacy and family support organization dedicated to supporting and empowering patients and their families affected by Niemann-Pick Disease (NPC & ASMD), through education, collaboration, and research. Like any organization, it's the people that make it special. And among the first people you'll meet at NNPDF is Family Services Manager, Laurie Turner. Whether you are new to NPC or have been part of the community for years, Laurie always has programs, resources and ideas, and an understanding way to brighten your day. Meet Laurie!



Laurie Turner  
Family Services Manager,  
National Nieman-Pick Disease Foundation

### *What is your role at NNPDF?*

I am the Family Services Manager here at NNPDF. I have worked with rare disease families for over 20 years and have been with the Foundation for almost five years. I am the main point of contact for families as they navigate NPC. I offer support and provide resources at all phases of NPC. I spend a lot of my time talking with families on the phone— helping them find and secure resources locally, connecting them with another family with NPC, connecting them with medical professionals— helping wherever it is needed most. I work closely with Lori and the team at Cyclo to ensure NPC families are educated and informed of potential experimental treatment options.

### *What is helpful for caregivers facing a new diagnosis of NPC to know?*

You are not alone on this journey; we have a great network of others that understand and are available to help you along the way. I often remind newly diagnosed caregivers that they are doing a good job, learning, navigating, and advocating— sometimes they don't recognize that they are doing their best and it is helpful to have someone else acknowledge this. I am also a strong believer in empowering NPC families— helping them find information and resources that will best help their loved one with NPC— learn as much as you can so you can make best choices for your family.



### *Meet Laurie Turner, cont.*

#### *What do you love most about your work with the NPC community?*

The families— they are the best, and I am so thankful that they are willing to share a small part of their lives with me. They provide my purpose and inspiration each day.

#### *How can people learn more about the support offered through NNPdf?*

- Enrolling for membership with NNPdf— membership is complimentary and ensures you will be kept up to date on all important news and information: [www.nnpdf.org](http://www.nnpdf.org)
- Attend our Annual Family and Medical Conference: <https://www.nnpdfconf.org/>
- Follow NNPdf on social media: [@NNPDF](https://twitter.com/NNPDF)
- Connect with me directly: [lturner@nnpdf.org](mailto:lturner@nnpdf.org) or 920-542-4038



NATIONAL NIEMANN PICK DISEASE FOUNDATION