



Hello from Cyclo Therapeutics!

We hope your new year is off to a great start. We are tremendously grateful for your connection with us, and for the engagement and support you have graciously shown to us throughout this year and beyond. Included in this newsletter is a message from our CEO, Scott Fine. Scott shares an update on our ongoing Phase 3 study, TransportNPC™ and a recap of key activities delivered last year. As always, please reach out to me with any questions.

-Lori and the Cyclo team

Lori McKenna Gorski
Global Head of Patient Advocacy

TransportNPC

Newsletter

To our NPC Community,

I'd like to reflect upon the meaningful progress we have made on behalf of those living with Niemann-Pick Disease Type C (NPC), and to express our gratitude for your engagement along this important journey.

Our study, [TransportNPC™ \(Intravenous 2-hydroxypropyl-β-cyclodextrin\)](#), is the most extensive Phase 3 study underway to evaluate the safety and efficacy of a potential disease modifying therapy to treat NPC. With the support of physicians and the patient community around the world, I am pleased to share we have dosed patients in every region of the world where sites are actively recruiting, including in the United States. We expect to complete enrollment by the end of 2023. Our energy is now on increasing and activating the number of clinical trial sites to help those who are interested in accessing our study in the U.S. and abroad.

In addition to actively enrolling this study, we have achieved a great deal more in 2022 with your support:

- ✓ We established an NPC Global Steering Committee of key physician experts from around the world to advise us on our work.
- ✓ In the U.S., we established a Patient Advisory Board to provide feedback to inform our work. We convened a similar family working group in the U.K. in October, thanks to [NPUK](#) and held a working group at the [NNPDF](#) annual meeting.
- ✓ We published Phase 1 data for Trappsol® Cyclo™ in the Journal of the Society for Inherited Metabolic Disorders, Molecular Genetics and Metabolism.
- ✓ We connected with the community at many key events, including annual meetings for NNPDF/INPDA, Australian NPC Foundation, and NPUK.



N. Scott Fine
Chief Executive Officer



TransportNPC

Newsletter

To our NPC Community, continued.

- ✓ For physicians, we presented global scientific congresses including WORLD February 2022 World Orphan Drug Congress, the Latin American Course on Lysosomal Storage Diseases, and the Genetics Rare Immune Disorders Symposium (GRIDS) with Dr. Caroline Hastings as invited speaker.
- ✓ We expanded our clinical strategy focused on neurodegenerative diseases and commenced our Phase 2b study of Trappsol® Cyclo™ in Alzheimer's Disease, further extending the promise of this investigational therapy to become a targeted treatment addressing complex disease processes caused by disrupted lipid pathways and support unmet medical needs in diseases and patient communities currently underserved.

In 2009, we provided the first patients with hydroxypropyl-beta-cyclodextrin to address the symptoms of NPC. Our work has only intensified. Every patient and family living with NPC deserves access to safe and effective therapies, and we are committed to doing our part to bring an approved therapy to fruition.

Looking ahead toward 2023, we have and will continue to focus the majority of our resources, both human and capital to the TransportNPC™ program for the benefit of the global NPC community.

Respectfully and with gratitude,

Scott Fine, CEO

Breaking News

Cyclo Therapeutics will have a poster presentation at the Society for Inherited Metabolic Disorders (<http://www.simd.org/>) regarding TransportNPC™. SIMD is a great meeting and opportunity for us to share our NPC story with clinicians and researchers devoted to inherited metabolic diseases.

TransportNPC→ Newsletter

Takeaways from the UK

Back in October, our team attended the Annual Scientific and Patient Conference on Niemann-Pick Disease, brilliantly organized by [Niemann-Pick UK \(NPUK\)](#). The meeting included sessions regarding science and innovation, progress in the patient registry ([International Niemann-Pick Disease Registry \(INPDR\)](#)) and sessions focused on supporting the patient community.



Here is just some of what I took home with me:

- The [INPDR](#) has made major strides, doubling in size over the past two years. Special thanks to families around the world who have included prospective and retrospective data to strengthen this crucial tool. And welcome Serbia to the registry!
- The interactive scientific workshop included nearly 20 presentations including newborn screening, research updates and clinical developments in Niemann-Pick Disease. It is inspiring to see the mix of presenters, including luminaries like Dr. Caroline Hastings alongside Ph.D. students and other future leaders. As some rare diseases risk not having a pipeline of devoted thought leaders, the Niemann-Pick community shows great commitment to supporting the work of young researchers. Together we must continue to foster curiosity about Niemann-Pick and all rare diseases. Kudos to NPUK for showing us the way. We published Phase 1 data for Trappsol® Cyclo™ in the Journal of the Society for Inherited Metabolic Disorders, Molecular Genetics and Metabolism.
- Advice from actual patients and caregivers to the world: Any communications to patients and caregivers must be presented in language that speaks to them. Don't repurpose scientific or investor presentations. Start from scratch.
- The clock is ticking. Niemann-Pick Disease Type C is rare, progressive, and fatal. Patients and their families urgently need effective treatment options, and we must work together to advance clinical studies.

It is a privilege to participate in meetings such as these. I remain eternally grateful to the heroic patients and families who share their journeys with us, to help the greater good. We hear you, and we will work harder having met you.

-Lori McKenna Gorski, Patient Advocacy

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A Year in Photos

