

Therapeutic Update



Dear NNPfD Community,

We are pleased to announce that we have expanded the team at Cyclo Therapeutics to include our new Chief Medical Officer, Lise Lund Kjems, MD, PhD. Among her many responsibilities, Lise will be focused on our late-stage clinical program, Trappsol® Cyclo™ and our ongoing Phase 3 TransportNPC™ study for the treatment of Niemann-Pick Disease Type C. Lise will be participating in community events and is looking forward to engaging with you. For now, we thought the best way to introduce her to all of you, is through this interview.

Tell us about your background:

I am from Denmark and received both my MD and PhD from the University of Copenhagen Medical Faculty. I live in the United States and have devoted my professional career to bring treatments to market for chronic, rare and ultra-rare diseases with high unmet medical needs. Most recently, I lead the development program for an ultra-rare pediatric liver disease, and the drug recently received approval in both the U.S. and Europe. In my role, I truly value being able to connect with the patient community as the most profound, impactful, efficient way to learn about a disease.



Why did you want to join Cyclo?

I am drawn to roles where I believe I can make a real difference and have an impact on the lives of patients and their families. As the community knows better than anyone, there is an urgent need for approved therapies to treat NPC. I believe I can play an important role in advancing our Phase 3 trial, TransportNPC™, rapidly. This is my first priority and what inspired me to take on this role. Additionally, those who I have met at Cyclo all share an intensity and commitment to improving the lives of those with NPC. We each bring complimentary skills and backgrounds, and a unified focus on our work at hand. I will continue to collaborate closely with Sharon Hrynkow, our Chief Scientific Officer, who has led so much of the drug development program over 6 years, and with Gerry Cox who is so well known to the Niemann Pick Community, will remain actively involved in our program and as a member of our Scientific Advisory Board.

Can you share an update with us on TransportNPC™?

Since the study is now underway, we cannot provide interim updates with you. What I can say is we are actively enrolling in sites in the U.S. and we will update clinicaltrials.gov as new site locations become active. If you are interested in enrolling and do not see a site listed near you just yet, please have your treating physician get in touch with us. As a reminder, in the TransportNPC™ study, we plan to enroll 93 pediatric and adult patients with NPC1 around the world. Eligible patients will be

randomized 2:1 to receive investigational drug (Trappsol® Cyclo™) or placebo. The study will last 96 weeks and include an interim analysis at 48 weeks. We understand that 96 weeks can feel like a long time. If we see definitive results at the 48-week mark, that may enable us to accelerate our timelines and switch those from placebo onto study drug earlier than planned.

What inspires you?

Science and innovation that translate into novel therapies and experience the differences when available to patients and subjects at risk and the power of collaboration.

What do you like to do for fun/Where is your happy place?

Outdoor activities such as walking, biking, gardening. I enjoy classical music and cooking. I love to go off the beaten path when travelling and learn about new customs and cultures. My family is my happy place.

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