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Global Head of Patient Advocacy

Hello!

It's a pleasure to connect with you with another edition of our newsletter. I am happy to share a new feature with you called "Powerful Patient Advocates." It has always been admirable to see the connectivity in the NPC community, with medical professionals, with industry, and with each other. I hope you find these features both informative and inspirational, and a reminder that you are not alone in your journey of NPC. In this first feature, I am pleased to introduce you to Barbara Lazarus. I am sure many of you know Barbara already from her advocacy work on behalf of both her two adult sons and the entire NPC community, but I hope everyone can learn something new from her experience and her advice. Do you have ideas of other powerful advocates we should feature? Please reach out to lori.gorski@cyclodex.com and let us know.

We are also looking forward to connecting in person with many of you at the NNPDF meeting this summer in Orlando. This will be a great moment to come together toward our common goals-- to advance research, understanding, awareness and support for Niemann Pick Disease. And ultimately, to have approved treatments available for all who need them.

Enjoy your spring.

- Lori and the team at Cyclo Therapeutics



Meet Barbara Lazarus

Tell me about your background, how you became part of the NPC world.

I worked for many years as an educational and clinical speech pathologist, using my knowledge and voice to support and treat children and to advocate on their behalf. My husband and I have two adult sons, Daniel and David. Daniel for many years had unexplained physical and memory issues, and David suffered from psychiatric issues that were severe. While presenting with different experiences and symptoms, both Daniel and David eventually were both diagnosed by an astute movement specialist who put all the pieces together for us. So, in 2017, we received our NPC diagnosis. Daniel was 30 and David was 28.



Gary and Barbara, with their sons David (left) and Daniel (right)

You are an outspoken advocate and participate in many events for the NPC community. Why do you do it?

I want to advocate for ALL of those with NPC and their families. But my launching point is based on my own experience. My sons got their diagnosis much later in life bringing challenges that were different from the pediatric journey. Imagine a six-foot-tall adult man on a pediatric exam table. And as parents of adult children, we did not and do not have complete control over caregiving decisions. Finally, the rest of our own lives were not going to be what my recently retired husband and I expected. So, if you combine the experience of two adult sons having a new diagnosis of NPC, with my professional work as a speech therapist and advocate,

the most natural path for me to pursue was to focus on supporting the adult NPC patient experience. This is who I am, and I believe that if you don't speak up you don't get action. I admire all advocates. There are a lot of things I am not. But for me, my voice is my advocacy.

In addition to treatment options, what else do you want for those with NPC? For their caregivers?

Ultimately, I want what every single NPC family wants which is a cure. But, in the meantime, I want options - options for research, for treatment and for support. I feel the best way to achieve that is by working together as a community, presenting a united front and accepting ALL potential treatments in the hopes of making this a chronic disease which can be managed as opposed to the fate we have been handed.



Meet Barbara Lazarus cont.

What was it like participating in a clinical trial?

When Daniel and David were diagnosed, we were all stunned. We were a family in crisis. We looked at our options, and there was one study that was available to us. So, we sat down and together we weighed the pros and the cons. As we sat down as a family, we discussed could there be side effects, could there be benefits, what would life look like, how much time would it take out of our lives? We spoke with the clinical trial organizers, and we asked every question we could think of – logistics, travel, time, what actual medical procedures would happen, etc. We found the conversations comforting and reassuring. We are glad we decided to participate, but I know it is stressful and a personal decision. My recommendation is to ask every question you can think of, talk to others who have participated and decide once you know in totality what the family experience would be like – physical, emotional and financial.

What advice would you give to a family just receiving an NPC diagnosis?

There's hope. There is a path to treatments. The best thing you can do is to open yourself up to others who have been through this experience. You are not alone. Ask your questions, speak up. Seek out support and accept it.

What effect did the COVID pandemic have on you?

I don't like to sit still and lock down was HARD. One day, I was sitting on my couch and NNPDF sent out a notice for volunteers. I raised my hand. I reached out to Joslyn and Laurie and I said, 'this is my background, this is what I have to offer – how can I help?' So I guess there was a little good that came out of a global pandemic!

Where is your happy place? Your sons' happy place?

My Daniel is a movie buff and loves action movies. David loves sports and is a huge Yankees fan like the rest of our family. But for me, my happy place is anyplace where I can put my feet in the water with a good book in hand. I can get lost in a good book.

We need your voice!

Perspectives from the Niemann-Pick disease community play a central role in every area of our work. We are working to enroll NPC patients in our Phase 3 Program called TransportNPC™, which is currently the most advanced clinical research program in NPC in the world. To help support and guide our work in the months and years ahead, we are putting the finishing touches on a new NPC Patient and Caregiver Advisory Board that will help to bring our team a broad range of perspectives and opinions that will support our work, while making sure that our work is aligned with the most urgent needs of the community. Patients and caregivers who join our board will have the opportunity to join in a two-way interaction that is designed to advance understanding of the burden of NPC, the needs of the community and the best ways to advance research. Through these discussions, Cyclo Therapeutics will collect feedback and use this guidance to plan for our continuing efforts to develop a treatment for NPC. The Board will be comprised of 6-8 NPC disease advocates based in the United States, including both patients and caregivers. These members will be invited to work with many different members of the Cyclo Therapeutics team to build a better understanding of our mutual goals and the best ways to advance research moving forward.

If interested, please reach out to:

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TransportNPC