University of Rochester NMO-Health Index Study

You can watch the video of this podcast at: youtu.be/0BRdMNCDNMw

[00:00:00] **Krissy Dilger:** "ABCs of NMOSD" is an education podcast series to share knowledge about neuromyelitis optica spectrum disorder, or NMOSD, a rare neuroimmune disorder that preferentially causes inflammation in the optic nerves and spinal cord. "ABCs of NMOSD" is hosted by SRNA, the Siegel Rare Neuroimmune Association. SRNA is a nonprofit focused on support, education, and research of rare neuroimmune disorders. You can learn more about us on our website at wearesrna.org.

[00:00:51] Hello and welcome. My name is Krissy Dilger, and I moderated this episode titled, "University of Rochester NMO-Health Index Study." Today I am pleased to be joined by Matt Rathbun and Charlotte Engebrecht. Matt Rathbun graduated from Nazareth University in May of 2025 and is currently pursuing his Master of Public Health degree at Nazareth University. At the University of Rochester Center for Health and Technology, he works as a Human Subject Research Specialist where he coordinates translational research studies focused on the living experiences of individuals living with rare diseases.

[00:01:34] Charlotte Engebrecht is a graduate of Hobart and William Smith Colleges and a current Master of Science in Clinical Investigations student at the University of Rochester. She serves as a Clinical Trials Project Specialist at the University of Rochester Center for Health and Technology, where her work centers on the development and validation of patient-reported outcome measures for rare diseases.

[00:02:00] You can view their full bios in the podcast description. This episode is made possible in part by the generous support of Amgen; Alexion, AstraZeneca Rare Disease; Genentech; and UCB.

[00:02:15] Thank you both so much for joining me today. I appreciate you

[00:02:19] coming to talk about your study that I'm sure people are going to be very interested in. So, can you start off by providing a brief explanation of what your study is about and the background on how it came to be?

[00:02:35] **Matthew Rathbun, BA:** Sure. So, our study is called the Neuromyelitis Optica Spectrum Disorder-Health Index Study. We are developing a patient-reported outcome measure that's designed specifically for individuals who've been diagnosed with NMOSD.

[00:02:52] So this is a survey that will be used in future clinical trials and regular patient care to measure how patients are feeling and functioning in response to therapeutic interventions. And so, in regard to how this study came to be, because NMOSD is such a rare condition, the way that health is measured in clinical trials is often adapted from more common disorders such as multiple sclerosis.

[00:03:26] And the surveys and patient-reported outcome measures that are used in these trials aren't specific to the needs and unique experiences of individuals with NMOSD. And we decided to conduct a study to develop a survey that's specifically designed for NMOSD patients.

[00:03:52] **Krissy Dilger:** Awesome. That's really interesting. And I think that's a common thread that we get. Because NMOSD and other disorders we advocate for at SRNA are so rare, a lot of the information that is available is actually for MS or other disorders and diseases. So, it's really awesome that this is something you've chosen to focus on and develop.

[00:04:23] **Matthew Rathbun, BA:** Yeah. Thank you. We appreciate it and fully agree. It's definitely very important to focus on the unique experiences of individuals with rare diseases.

[00:04:38] **Krissy Dilger:** So, can you talk about what the purpose of this study is?

[00:04:43] **Charlotte Engebrecht, BS:** Yeah, I'd be happy to talk about it. Imagine you're testing a new medication and you're only looking at lab numbers, but you never actually ask the patient if they're feeling better. Using these surveys can help address that. So, it's kind of like a way of checking in directly with the patients to see if a treatment is making a real difference in their lives and not just on paper. So, it's very important because it brings the patient voice into clinical trials, when historically that's not been a component.

[00:05:16] When new drugs are tested, the FDA actually looks for this kind of information to help decide if a treatment should be approved. So, these surveys ask patients directly about their symptoms and how it impacts their daily life. And we think it's very important because patients are really the only ones who can fully describe how they feel.

[00:05:38] And without using these surveys, we might miss whether a treatment actually makes a patient feel better, and that's the whole point. So, we want to make sure that we address that, which is why it's very important to include them.

[00:05:52] **Krissy Dilger:** That's really interesting. And I can relate to that feeling of feeling like only the medication is what's in focus instead of how it's making the patient feel. That's, I think, something that is really important, like you said, and I'm interested to hear more to see what you've found. Can you explain how your study is set up?

[00:06:20] **Charlotte Engebrecht, BS:** Yeah. So, this is a multi-step process to really make sure we capture or truly reflect what the patient's experience is. So first we start off and talk with patients directly, ask some open-ended questions about the symptoms they experience and how it impacts them.

[00:06:39] And this is really important because it helps us understand, like you were saying just before, the full picture of the patient, especially in the patient's own words and not just what doctors or clinicians may think is important. We also get the patient perspective. And from those interviews, we take all of those symptoms that were identified and turn them into an online survey. So here we want to send it out to a large group of people with the same condition.

[00:07:08] And this helps us make sure that the symptoms aren't just a few individuals' experiences, but actually common across the broader community. So based off of those results, we then can refine the survey and make sure that the symptoms that are included are reflective of the whole community and we're not having irrelevant questions in there that might increase some survey burden.

[00:07:31] After that, we go back to the patients again and we test the survey with them—this new version of it—and make sure that the questions are clear, it's easy to understand, nothing's redundant.

[00:07:43] We're really making sure we measure what matters to them and make sure we're not missing anything important. So, the fine-tuning of it. And then we have our last part of it, which is the reliability testing. So, we want to check the surveys and make sure that they're consistent over a short period of time. And that's really an essential part if it's going to be used in clinical trials to evaluate new treatments.

[00:08:08] We want to make sure that one of the assessments is reliable over time. So overall, at every step, patients are guiding the process of how we develop the survey to make sure that the final survey truly reflects their whole experience and it's not just based on some assumptions.

[00:08:27] **Krissy Dilger:** Very interesting. And can you talk about who is eligible to participate in this study?

[00:08:35] **Matthew Rathbun, BA:** Yeah, I could touch on that. So, we're currently recruiting for the second phase, which Charlotte touched on. And this is the large international survey. And so here the eligibility criteria would be anybody who is over 18 years old and has a diagnosis of NMOSD.

[00:08:59] And that's inclusive of individuals who are both aquaporin-4 positive and aquaporin-4 negative. And in terms of countries, we are accepting individuals from the US, Canada, European Union, United Kingdom, and Australia.

[00:09:25] **Charlotte Engebrecht, BS:** And to go off of Matt further, too, this is a completely anonymous survey, and I think for expected completion time, it's about 20 to 30 minutes to do the full survey. We have some basic demographic questions that are in there, and then also the direct NMOSD-specific symptom questions as well.

[00:09:49] **Krissy Dilger:** Great. You mentioned that your phase one portion of this study is complete. What can you tell us about the results from that phase? Are there any interesting findings so far that you are able to share?

[00:10:08] **Charlotte Engebrecht, BS:** Yeah, I think the main, or one of the biggest, takeaways from our results is that the condition affects multiple areas of health. It's not just physical symptoms. People frequently mentioned challenges with mobility, fatigue, vision problems, and pain. But they also talked about how these symptoms make it harder to participate in everyday activities and how it impacts their social life and relationships.

[00:10:38] Also their emotional health, anxiety was one symptom that was frequently mentioned as well. And I think this is very important because the research shows that the condition affects physical, emotional, and social aspects of quality of life.

[00:10:52] It's not just one domain or one segment. So, if we're only focusing on measuring the physical symptoms like some outcome measures or assessments in clinical trials, we're really missing the big picture and the full picture that there are also different components of their health that are affected. And Matt, I don't know if you want to add on?

[00:11:13] **Matthew Rathbun, BA:** Yeah. Just to build off of that. In our interviews, we talked to 15 individuals with NMOSD, and there were several areas of health that were experienced by every single person that we interviewed. And for example, these include things like limitations with mobility or walking, pain, and impaired sleep or daytime sleepiness.

[00:11:43] Numbness was also frequently mentioned. And so really, it's very severe multi-system impacts that individuals are experiencing. But it's also important to note that this is just a snapshot of these 15 individuals that we interviewed. And so, the whole point of this survey that we're launching for this next phase is to test the prevalence of these symptoms among a much larger group of NMOSD patients, which is really a crucial step in the research that we do.

[00:12:27] **Charlotte Engebrecht, BS:** And going off that, too, I just wanted to add and basically echo what Matt was saying. How it's very important to have a lot of individuals participate in this online survey that we're doing. Because then every person who participates can also eventually help make sure future treatments are evaluated based on what actually matters to the patients.

[00:12:52] Because we're having this exact phase to the larger group of people, making sure that it's actually reflective of the whole community. So, the more participants that we get, the greater the results could be and more reflective of the community.

[00:13:10] **Krissy Dilger:** Very interesting. Thank you so much for sharing, and I'm interested to see where this develops and goes from here as you progress into the later phases of the study. Which leads me to my final question: how can someone participate in this study?

[00:13:30] **Matthew Rathbun, BA:** Yeah, I can touch on that. So, the survey that is in this next phase is entirely online and it's completely anonymous as well. There's a link—I'm not sure if it'll be in the description of the podcast here—but there's a link that you can click on and it'll take you to our online survey, where you would be directed to our information letter, which has a really in-depth overview of our study and our goals with what we're trying to learn through this research.

[00:14:11] And then there are some brief demographic questions, as well as questions about your clinical history with NMOSD. And then lastly, it's specific questions about the symptoms that were identified during the first phase of interviews with NMOSD patients. And here you would essentially just rate the impact that each of these symptoms has on your daily life.

[00:14:44] **Krissy Dilger:** Awesome. Yes, we will include a link to the study, as you mentioned, in the podcast description. So, check there. You can also reach out to us at SRNA. Our email is info@wearesrna.org and we can point you in the right direction towards our friends at Rochester. Lots of ways to get involved. And can you clarify whether someone who's participated in the phase one portion can participate in the second phase?

[00:15:21] **Matthew Rathbun, BA:** Yeah.

[00:15:22] **Charlotte Engebrecht, BS:** Good question. I'm sorry, Matt. I was going to say, yes, they are able to participate in the previous phase as well. And then also the same thing goes as well, just because you participated in a phase—or if you didn't participate in the early phases—you are still allowed to participate in the later phases. So, it's an overall welcome. You can participate in any phase.

[00:15:43] **Krissy Dilger:** Awesome. Yeah, I'm excited to see what this study yields in terms of results. It seems like really important and interesting work.

[00:15:56] **Charlotte Engebrecht, BS:** Thank you, and thank you for allowing us to talk about it. We're always very happy to share our results and excited about our next steps.

[00:16:04] **Krissy Dilger:** Of course. Yeah. We'll have to have you back in however long it takes until the end so that we can hear about all the findings and what comes of it.

[00:16:15] **Matthew Rathbun, BA:** That'd be awesome.

[00:16:17] **Krissy Dilger:** Thank you both so much for joining me. And we'll put both of your email addresses also in the podcast description in case anyone has any questions about the study or how to participate. And thank you so much for your time.

[00:16:33] **Matthew Rathbun, BA:** Yeah. Thank you, Krissy.

[00:16:34] **Charlotte Engebrecht, BS:** Yeah, thank you. We enjoyed it.

[00:16:41] **Krissy Dilger:** Thank you to our "ABCs of NMOSD" sponsors: Amgen; Alexion, AstraZeneca Rare Disease; Genentech; and UCB. Amgen is focused on the discovery, development, and commercialization of medicines that address critical needs for people impacted by rare, autoimmune, and severe inflammatory diseases. They apply scientific expertise and courage to bring clinically meaningful therapies to patients. Amgen believes science and compassion must work together to transform lives.

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[00:18:12] UCB innovates and delivers solutions that make real improvements for people living with severe diseases. They partner with and listen to patients, caregivers, and stakeholders across the healthcare system to identify promising innovations that create valuable health solutions.