CIRCLES and SPHERES NMOSD Studies

You can view this presentation at: youtu.be/OZYWFs7Ygps

[00:00:00] **Roberta Pesce:** For our next talk, I'm joined by Jacinta Behne, executive director at the Guthy-Jackson Charitable Foundation, who will talk about the NMOSD CIRCLES and SPHERES studies. Hi Jacinta, good to see you again.

[00:00:18] **Jacinta Behne:** Hi, Roberta.

[00:00:18] **Roberta Pesce:** Over to you.

[00:00:20] **Jacinta Behne:** Great. Roberta, is my audio okay?

[00:00:22] **Roberta Pesce:** It is perfect.

[00:00:23] **Jacinta Behne:** Wonderful.

[00:00:24] **Roberta Pesce:** Yes, I can hear you loud and clear.

[00:00:25] **Jacinta Behne:** Thanks a lot, and thank you so much to the Siegel Rare Neuroimmune Association, or SRNA. It's such an honor to be here with you today for your rare neuroimmune symposium, and well, let's just, I've got a lot of information to share. I'm going to go at a fairly rapid click, and hope I don't talk too quickly. I'm Jacinta Behne, executive director of the Guthy-Jackson Charitable Foundation. Today I'm going to talk about two NMOSD studies. The first being CIRCLES, which is a study that we sponsored, and the second being SPHERES. One that's just in the process of launching and such. So let's proceed.

[00:01:09] The Guthy-Jackson Charitable Foundation, I'll give you a little background to start with for those of you to whom it's new. We started August 1st, 2008, going strong until now, and in the future. The cause for the foundation to be formed was Victoria Jackson and Bill Guthy, who had a high priority to learn something about this devastating disorder that their 15-year-old daughter, Ali, and this is public information, was stricken with, and they learned that it was NMO at the time. It was a rare autoimmune disorder, and there was very little known about it.

[00:01:53] So August 1 is the formal start date for the Guthy-Jackson Charitable Foundation in 2008. I've been with the organization since then, and it's been rewarding and at the same time challenging, and much work ahead. Our primary goal, the charter of our foundation is research. Our whole goal is to advance NMOSD knowledge, and I'll just kind of tick through some things that we knew quite early on, that we really needed to get focused on and forming, and one was what we called IPND, an international panel for the diagnosis of NMO. It was the case then, as sadly it still is now in some areas of the country and the world, hard to get a reliable, a correct diagnosis for NMO.

[00:02:45] Far too many of our patients who we talk to, some took 5, 10, 15 years to be diagnosed. The more recent patients who join our community tell us it's been sometimes just weeks, so we worked really hard on that and pulled together a panel of 20 researchers, global researchers from the states to Australia and back. And they worked for nearly 2 years, lit review of 250 research publications, and they came out with what's called the IPND criteria, which is an international consensus on diagnosis of NMO. By the way, many people will ask often, so what's the difference between NMO and NMOSD? Well actually there is no great difference unless you want to get down to the strict definition.

[00:03:33] NMOSD, the SD standing for spectrum disorder, took NMO from a just it's own disease, which was by then well differentiated from MS. We had initially been considered an MS variant, which we know now it very much stands on its own, just like MOG is in that process as well. So NMO, initially NMOSD. We'll still say NMO many times and neither is wrong. We also knew that we had to find an adjudication tool for relapses. When patients have their initial event or their first attack of course it's devastating.

[00:04:15] What we've also learned is from the time of diagnosis until, well until as long as they're carrying that disease forward and walking that path, the question is when. When will there be another relapse? Is there any way for me to know any early signs? How can my doctor know for sure that I'm having a relapse? There are times when I go to him and her and see them and say I think I am, I'm not sure I am. So we really had to work on something called the relapse navigator tool. This has taken several years, it's in its validation phase right now. We knew that we had to bring people together for international conferences and symposia, which is happening for us also at ECTRIMS, in 2 days, on the 12th, the day prior to ECTRIMS, and bringing people together early.

[00:05:08] We, as I said, August 1st, 2008, we began. That following November we had our first round table of bringing clinical researchers together. There were about 20 of them, and we acted quickly, and our founders asked them to work together and we would work to fund studies and ask them to please learn and study this disease. Patients always come first. Research is our primary goal. Obviously for the benefit of patients. So we have put a lot of time and resources into advocacy and resources. That could be an entirely different presentation all on its own. We count over 1,800 research publications. No, we didn't write them all, but members of our international clinical consortium, our CIRCLES study researchers who simply come to our conferences and have worked together have become quite prolific since 2008 in publishing. We're going to talk a little more in this study, or excuse me, this presentation about SPHERES, a longitudinal study.

[00:06:15] Yes, we're about science saving lives. We have funded around 103 studies, either direct funding, shared funding, providing resources that simply cannot be gotten any other way, and it has, we also created a ground swell of grown science so that we know what NMO is and what NMO is not. We've invested about $70 million since 2008 in funding research, and doing everything we can to bring people together to continue the conversation. We have an NMO biorepository and data repositories. I'll talk about that when we turn to CIRCLES. We have worked really, really hard to facilitate clinical trials with the three pharma organizations who we know. The result of that is in 2020 having three therapeutics on the shelf, and so grateful to those industry members.

[00:07:17] We formed an international clinical consortium because we knew that we don't know who all the neurologists are around the world who treat NMO, and we now have 112 members worldwide and 30 countries. I think it may have just turned to 31 this past week. Now let's turn to CIRCLES, which there have been at least 50, maybe more research projects, and as you see these photos, a wonderful, giving lady, who's willing to at one of our patient days we had a draw room set up every year, and here she is giving blood with a smile, and you'll see in the next slide where those specimens go. It's direct into a package, a container of vials that go into a minus 80 freezer at our biorepository at Covance in Greenfield, Indiana.

[00:08:10] What is CIRCLES? It's a collaborative international research and clinical longitudinal experience in NMO. That's a long title, thus the acronym, CIRCLES. It's a longitudinal-observational study protocol. We follow patients over a period of time, it's become an established and trusted patient study platform because frankly, we need to have that by that data repository in order to fuel the research so that we can learn more each day about NMO. There are over 1 million clinical data elements in our data bank, and the biospecimen and data collection together period was 2013 to 2020, 7 years with 15 academic centers. We had over 1,250 cases and respective controls who participated, and if you're one of them, bless you. We are so grateful.

[00:09:09] LabCorp and what is now Covance has supported the CIRCLES study sites from day 1. They manage our biospecimen storage. When researchers are requesting specimens, they ship those specimens in the United States and internationally. The University of Utah Data Coordinating Centers are clinical biospecimen database. For every specimen that's in the Covance site, there's a collocated piece of data, clinical data for an unidentified patients, identified simply by a barcode at the data coordinating center. They perform biostatistics for us, and research data mining studies. Then we have CIRCLES, and CIRCLES concluded in 2020. Now that's the collection phase. The requests for specimens and data of course is still very active, and CIRCLES is still very much alive, and we are now in the process of providing those resources for studies.

[00:10:24] The study sponsor for SPHERES, we've gone from CIRCLES into SPHERES is an organization called Corevitas. They were known as Corona. They recently became Corevitas, and SPHERES study is enrolling now. And the whole point of SPHERES is for patients, obviously, but the whole point is looking at therapeutic effectiveness, safety, and quality of life on behalf of the stakeholder community. Patients have an opportunity to be aware of the solution and a part of the solution. Corevitas has a well known expertise in the autoimmune space, and the studies informed by the CIRCLES experience, and we have a number of trusted partnerships among NMOSD patients, and key opinion leaders and clinicians who are serving as PIs now.

[00:11:20] So maybe you're going to be somebody who will be a part of SPHERES. If your doctor has asked you we hope you'll consider, and if they haven't you can ask, but just so you know, if you hear SPHERES that's what we're talking about. It's a unique collaborative environment. It's important to know it's regulatory grade, which means it's going to look at safety, efficacy, patient reported outcomes and relapses, and there's a wonderful economy of scale by working with a consortium of pharma partners for data elements and biospecimen acquisition once more. It's important to think about our patients from their own perspectives.

[00:12:00] I'd like to just drill down now into why and who we do this for, here we have a female patient who said before NMO I was a very active and happily employed mother and wife. Now I cannot work because of my disabilities and incontinence issues. I have to rely on special transportation services which are often unreliable. Costs of medicines and services are taking a big toll on my family. And if you're a patient who is attending this session, I can sort of envision some nodding going on, because this is a pretty standard response we hear from many, many patients. A male patient said as an NMO patient I am concerned that my condition will worsen to the point that I'll become a burden to my family physically, financially, and emotionally. I do not want to be alone and I do not want to be a burden to them or anyone. All, again a prolific statement for many of our patients.

[00:13:00] Since, oh goodness, over a decade we've had patients' stories, because we've learned every patient has his or her own story, and they've bee in text on our website. Many beautiful stories. This year we have taken them into hearing their voices, so this is what we call vignettes from patients and caregivers, and I thought it would be good to, instead of letting me do all the talking, hear from a caregiver and how their path at NMO has evolved, and I'll let him introduce himself and his daughter.

[00:13:40] **Roberta Pesce:** Jacinta, I don't think we can hear it, the audio, perhaps because...

[00:13:59] **Jacinta Behne:** I may not have clicked share audio, is that's what's going on you think?

[00:14:05] **Roberta Pesce:** I don't know. Or maybe because it's streaming in your headphones. I'm not sure.

[00:14:09] **Jacinta Behne:** I don't know. Okay, well let me go ahead and just...

[00:14:13] **Roberta Pesce:** Sorry about that.

[00:14:13] **Jacinta Behne:** I'm so sorry. What I will do, this particular vignette is available on our web site, and it's a beautiful story in the words of the father, Donald Campbell, about his daughter, Leah. Who in 2000, no it was late 1900s, not late 1800s, late 1999 or '89, I'm sorry, I'm not remembering the exact year. But I want to say it's in '99, she came down with a disease that simply couldn't be identified, and it took 18 years for Leah to have the correct diagnosis. In that period of time she became blind, a paraplegic for a while.

[00:15:03] She has regained some movement, and they're a beautiful family and together, and Donald talks about how his wife and Leah are his heroes now. They have moved forward and embraced life. He's become a great cook, driver of a van, a wheelchair guide and such, and oh, he also does laundry and dishes. So please all, if you'd like to just send me an e-mail or through info at, and we can send you the URL to this beautiful story of this father and daughter. And with that, let's go onto the next page. So where are we?

[00:15:52] Well, this is a call to action for patients and caregivers. How can you help? Because the one thing we've learned with NMO patients and caregivers is we see that there's a lot going on. What can we do to help? Well some ways to help is simply join us on social media, continue to get the word out with NMO. Most especially to help others who were recently diagnosed so they don't feel alone. No one should feel alone with this diagnosis. You can tell your own story in our vignettes program, and again, I'll give you the URL to go to do that, to see how you can accomplish that.

[00:16:38] Please, when we ask for participation in NMO surveys, we hope you'll respond. They're patient reported outcomes, they fuel our research, they fuel what we know and really need to hear from patients. Caregivers, what their daily experiences are, all manner of things, because ultimately the ultimate functional cure for NMO is in every patient and their caregivers. So please, please help us with that. Participate in NMO events as you can. There are regional patient days around the country. If you're near one you might participate in that. Numerous support groups that are going on, and also of course our annual NMO patient day, which we are looking forward to holding a face-to-face one. We'd love for that to happen in 2022, stay tuned. And then please encourage donations to help fund our research. It's how we've been able to get where we are right now. With that I want to thank you, I want to invite questions. I know that Roberta will help us with that, but please send any questions you have regarding things I've spoken about today directly, simply to Info@ GuthyJacksonFoundation.org, and I'll take it back to you, Roberta. Thank you so much.

[00:18:08] **Roberta Pesce:** Yes, thank you, Jacinta, so much, and for everyone who's watching, we have found Donald's story on your website and we have shared it with you in the chat so they can all look back at it.

[00:18:19] **Jacinta Behne:** Thank you.

[00:18:19] **Roberta Pesce:** Yeah, absolutely.

[00:18:21] **Jacinta Behne:** That's wonderful.

[00:18:23] **Roberta Pesce:** We got a question. Do I have to have access to my medical records to participate in either of the studies?

[00:18:31] **Jacinta Behne:** Oh, that's such a great question. The answer is your clinician needs to have access. It's through his medical records, because you'll be seeing your clinician in clinic, he'll be recording, asking some questions, kind of the same normal things that happen when you have your clinical exams, and he will be actually entering or his nurse will enter those data directly into the study. So if your doctor hasn't asked you yet about participating you might simply ask him, or if you know your clinician would like to participate, please ask them to reach out to me and we'll do everything we can to make that happen. It would be wonderful.

[00:19:23] **Roberta Pesce:** Perfect. Yes, thank you so much. And another one is where to find upcoming events and research?

[00:19:29] **Jacinta Behne:** Okay, great. Oh, good question. Upcoming events will be always on our home page, and right now we know that there will be a, patient day as an example, in March. Pandemic allowing, it will be, we're hoping and planning that it will be face-to-face. It will also be virtual, and it will be streamed internationally, so that information you'll find on our home page. I think it will be in the matter over the next few weeks, and upcoming research, generally research you're going to hear about the studies and the publications after the research is done. We can always tell you what's going on.

[00:20:16] One good way is to attend our break out sessions, which we have at least one a month, sometimes more. And the topics of those break out sessions are always prompted by what the patients are asking. Two wonderful people on our patient advocacy team, our director, Lisa McDaniel, her associate, her coordinator is Corey Wolf, and if you can capture their e-mail addresses, again, through info at or as you go through the website. I will tell you this folks, we know our website has got a lot of information, and we're going through that right now to try to even better streamline it without cutting out too much. So please, social networking is a good place to go to ask a message to Lisa or Corey, or our website. We'll always answer questions, we don't let them just sort of fall by the way side. I hope that helps.

[00:21:19] **Roberta Pesce:** Yes, absolutely. That was a great answer. You gave a lot of good information about where to find the information.

[00:21:27] **Jacinta Behne:** Yeah, sure.

[00:21:27] **Roberta Pesce:** Well, Jacinta, I think we've reached the end of our talk.

[00:21:31] **Jacinta Behne:** Great.

[00:21:31] **Roberta Pesce:** Thank you so much for your time.

[00:21:33] **Jacinta Behne:** Thank you.

[00:21:33] **Roberta Pesce:** I really appreciate it. It was good seeing you again.

[00:21:36] **Jacinta Behne:** Great, Roberta.

[00:21:36] **Roberta Pesce:** Thank you for all you do.

[00:21:38] **Jacinta Behne:** Thanks, and to those of you patients and caregivers out there, and your clinicians, we're so grateful to you, because we couldn't do what we do without you. Thanks a lot. Bye.

[00:21:50] **Roberta Pesce:** Thank you, Jacinta. Bye.