Know RND and the CORE TM Registry

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[00:00:05] **Dr. Carlos A. Pardo:** It is my great pleasure to introduce the next speaker. Dr. Kyle Blackburn from UT Southwestern. Kyle is the new generation of neurologists who are going to be taking care of neuroimmunological disorders and it is a great pleasure to introduce Kyle, because Kyle is one of our former SRNA fellows and has been very proactive at UT Southwestern to keep working and when Dr. Ben Greenberg retires, I don't know if he's going to retire, Dr. Kyle is coming and basically continuing the leadership of working on rare neuroimmunological disorders. Kyle, thank you.

[00:00:57] **Dr. Kyle Blackburn:** Thank you. Dr. Pardo. And it's a pleasure to be back here again. I think the last time we did this in person was three years ago, so it's always great. Just to remind everyone, Ben Greenberg is not retiring anytime soon. We've still got a ways to go.

[00:01:11] **Dr. Carlos A. Pardo:** All right.

[00:01:11] **Dr. Kyle Blackburn:** All right, I've got it now. So, I'm going talk about two projects that the SRNA has been supporting me on for the last few years. And this first one is a project that I've labeled, Know RND. Mainly because you need abbreviations and some of your IRB protocols. So, I needed some. So, this project is really born out of the idea of learning about how much knowledge people with rare neuroimmune disorders have about their condition and I just want to explain how I see complications with knowledge before we get into that. So, I think everyone here, in an ideal world would have received their diagnosis in a very streamlined fashion and I did not know that George Clooney was going to come up during our sexual health talk that was not planned.

[00:02:09] So, it would be ideal for your physician or your provider to review your symptoms, know exactly what's going on, perform the appropriate test and make a diagnosis. In this case we're going to use acute flaccid myelitis as the example. That would be the ideal situation and sometimes healthcare does work seamlessly like that. But unfortunately for many of you, your road looks like this.

[00:02:35] You go to multiple doctors, you get told multiple things, you're probably the number of people that I've heard turned away from the emergency room with transverse myelitis is shocking. But they're told multiple different things. It looks like I've got some stuff I need to click through. There are several MRI scans, repeated

MRI's over time. There is, I think we've heard it. It seems like all of your blood is gone. It's not possible that you could give more. There's multiple spinal taps and still you don't understand what your condition is and sometimes it takes coming to a place that's many, many miles from you to do that.

[00:03:15] And for unfortunately for some people, they never truly get an understanding and those are just the factors specific to healthcare. There are of course a lot of other barriers that can lead someone to have a poor understanding of their condition even with the correct diagnoses and not the least of those that the condition is rare. Of course, there can be certainly if you are in health care, you might have a little more of a leg up.

[00:03:46] So, there can be factors specific to your background and education and then in today's world, what's available online maybe outdated, like the one third, one third, one third thing that we've heard several times or just misinformation that were also up against that, but all of these are factors that could lead someone not to understand their condition and that's problematic because really understanding your health condition and having some baseline knowledge about the disease itself and what is underlying it helps people manage the disease and it helps prevent complications.

[00:04:45] So, if we apply diabetes as an example, if you don't have an understanding that diabetes is a disease of blood sugar, you may have a harder time understanding why you need this insulin. You don't understand that there are complications of diabetes. You may not understand that there are renal and neurological complications that could happen. So, in those instances that there is a lot of data to show in more common conditions that a good understanding of the disease has a firm basis in how you adhere to treatment and how you do the preventive care that's needed to prevent complications. And I think that's likely true of our diseases as well that we're covering today. So, to bridge this gap and I think a lot of this has been spurned from conversations with people over the years. There was a desire to learn how much does the community that SRNA serves, how much knowledge do they have about their condition?

[00:05:32] And that's a tough gap to fill because it is a very large group of people with a large group of different disorders and there really was no data on this at the time. So, we decided to embark and try to do that. So, this is a general outline of how the project will go. So, there is step one which is, you have to develop a measure and in research it is not good enough just to say I made a test. I wrote it. It does have to go through a couple of rounds of edits. People have to agree on content. There have to be certain domains that are agreed upon and beyond that there also has to be some degree of what we call validation and we've gone through that step. The second step is actually pilot testing.

[00:06:22] So, we're currently in this phase where we are testing the measure in a small number of people and I have chosen my own patients and I've specifically chosen patients who are not afraid to push back on ideas or talk or have a frank conversation with me about something because I wanted people that would speak up and say, hey, there need to be changes here that will refine the measure. And then in the last step there will actually be a dissemination of this measure to actually learn amongst a sample of people what the general knowledge is about these rare neuroimmune disorders amongst the community. So, step one is measure development as I was talking about. So, we have a team that is primarily composed of clinicians at UT Southwestern, but we have a neuropsychologist that's participating as well.

[00:07:10] And then we've also gotten feedback from members of the SRNA. These are the domains that the test covers. This may change over time as we adapt in the other phases, but we've got a very basic understanding of neuroanatomy, so the different parts of the nervous system symptoms that people may experience. It's a few questions about the cause or the understanding of these conditions from kind of the what's causing them and then, I think you heard Dr. Greenberg's talk yesterday about relapses and how sometimes that's not a straightforward understanding and that's certainly an area where we see a lot of confusion. So, we have several questions about relapses and treatment of the condition. So, we do ask people to also complete some information about educational background, about when they were diagnosed, are they still following factors that could influence their understanding of their condition.

[00:08:11] And then we're using... we have a measure of health literacy. So, health literacy is an individual's ability to understand health information. Whether it be information given to them by a doctor or from their insurance etcetera. And health literacy certainly would be a major thing that could correlate with the knowledge of your condition. So, we're currently at step two and we've actually planned to do this in five patients and again these are my clinic patients so they're all adults. So far, we've completed four out of five people somehow just in my clinic time, one transverse myelitis patient that has not landed in my lap. But we've had others with NMO and MOGAD participate. So, what we've done with each of those individuals is they've taken the test as if anyone else would online and then they have sat down with me for about 30-45 minutes afterward and we've just discussed how it went.

[00:09:12] We go through each question I asked a detailed assessment about wording, different ways that it could be assessed and well like I said, we're just waiting to do one more and then we're going to revise the measure further and send it for another round of comments and then we will move on to step three. And step three is where we actually will open up participation. During COVID we have gotten the ability to do consents in virtual form which makes it a lot easier for people that want to participate that may not visit UT Southwestern. So, we are excited to say that we will be able to open this through what's called RedCap which is just a database software that's available at most medical centers and it should be fairly easy for people to consent and then take the measure once it's available. And after that we'll be able to take a look at the measure and gain an understanding of how well the measure performs and learn a little bit more about the knowledge about condition specific to condition in the community.

[00:10:22] So that's the first project that I'm working on and then we're going to talk about one that's been going on for a little longer and that's the CORE TM registry. So, a registry is basically just a compilation of data, and it can be collected in a myriad of ways. And this is a project that I've been working on since first being introduced to the Transverse Myelitis Association. So, to explain a little bit about why we undertook this project, much of the information out there about transverse myelitis and many of these rare conditions and that looks at the outcomes that people experience, has really focused on measures that rely heavily on motor function. So, on the right, I actually show you what's the modified Rankin scale, which is actually cited in one of the major papers that looked at transverse myelitis and outcomes recently. The modified Rankin scale is a stroke scale and it does rely heavily on motor features, although people have tried to adapt it to include other domains to capture outcomes, but because it really focuses on motor and mobility status, I really feel that it's an imperfect measure and it doesn't measure all of the other symptoms that we've spent many hours talking about and during the symposium and I'm not even listing all of the potential ones there that we cover.

[00:11:49] So, there's really is a need to get creative about how we collect outcomes for patients with myelitis. And this registry certainly presents an opportunity to do that. And I went blank. Okay, there we go. So, this is how the study was designed. So, we wanted, again, we're trying to enroll people from... that were really as many people as we can. So, we've tried to be flexible, but we also wanted to focus on being able to confirm the diagnosis and one of our aims was also to look at the diagnostic accuracy. Again, what people are being told might be transverse myelitis as you've heard there are many people that may be misdiagnosed and have another condition like a stroke for example. So, we certainly enroll people, people consent right now we're using an old school paper consent process but still doing it through online. We have people send their MRI images and their medical records and we really want detailed medical records for many people that's going to come from a hospital stay from when they were diagnosed.

[00:13:01] And then once we have those, we have people complete surveys that's how the study was designed, and this is where we are. So, in research it's normal to have a certain amount of attrition. But this study has certainly had some barriers. Before last week we've had about 110 people email us expressing interest in this study. Again, it's normal for some people to not follow up on that. But we've only been able to get it and the numbers actually around 20 consented. I went back and did a recount like the Florida election years ago. And we actually have now enrolled 13 people that actually completed study procedures. So, we're a little bit lower than we want to be three years out. A little bit of information we've primarily gotten information on adults. This PDDS score is a... it's a disease step score that we've modified. It's primarily using multiple sclerosis outcomes for patients to report. And a four is an individual that's using a late CAIN status. That's people whenever they were at their lowest point.

[00:14:14] And then a one implies that someone has some symptoms usually sensory symptoms but has otherwise had good motor recovery. The immunotherapies that are commonly used were steroids and then a few people did receive IVIG and plasma exchange. So again, we've only been able to enroll 12 or complete 12 subjects to the finish line. And I've been trying to look at why that would be. And I really think the biggest block is in after people express interest or consent even is getting records. Medical records are a chore to obtain. I have certainly tried to step in and help people by filling out medical record requests myself and sending them in for this study. Even then I don't always get the records and even though it's a doctor asking.

[00:15:06] So, I can only imagine on the patient's side getting the records can be challenging. And then there's also some barriers that have been a number of people express interest that don't have an MRI scan or really can't track it down and that also can create barriers. So, these are some of the things and then there are probably another number of factors as well that have kept our numbers a little lower than we would like. So specific to this registry, every registry periodically does need to look at its procedures and evaluate new processes. And since this study has started, we have developed new measures, new consenting processes that UT Southwestern allows to embark on a virtual consent will make things a lot easier on both parties. I am strongly committed to lowering the burden of medical records if not removing the medical record requirement and relying on patient report to satisfy some of the measures that we're hoping to collect. I still think MRI that data and this is very critical.

[00:16:13] There may just be a subset of patients that we have to accept that won't have an MRI but if there are people that can get us an MRI scan, we're certainly going to make that an option because it does offer a lot of information to us. And as we look at ways to improve the consenting process and remove some of these barriers, I also think there's an opportunity to look at this critically and say, are there other outcomes other than things around sensory pain and about bladder function that we want to add? And in the time that I have created this registry, we've actually hired one of the foremost experts in multiple sclerosis registries at UT Southwestern. So, I'm very happy to say that I'm going to be contacting her to see if she can offer any advice on how to improve the registry, but also on future outcomes that we might be of interest. And there's even discussion that maybe we could expand this to other causes of myelopathy beyond just idiopathic TM to also capture a broadening phenotype of disease state. And that's all I really have for you all. Other than this picture of Kentucky, I always want to thank everyone for all the support and I'm happy to answer any questions. Sandy.

[00:17:43] **Sandy Siegel:** Thank you. Kyle, you raise a really, I think interesting question. And that is who do medical records belong to? Legally who do they belong to?

[00:17:57] **Dr. Kyle Blackburn:** There's actually recent legislation that would say you should have access to your medical records and actually can access them freely and they should be available to you. Now some of these records are older. So, I don't know that they're as accessible as they should be. But there has been a push to recognize that these records should be yours and that you should be able to access them. It's still not easy from what I've seen. And then even if you acquire them, sometimes even getting them to another medical center is another challenge.

[00:18:30] **Sandy Siegel:** Is that federal legislation?

[00:18:33] **Dr. Kyle Blackburn:** I think it's federal.

[00:18:34] **Sandy Siegel:** Okay. That's great. So, everybody needs to hear that because that is critically important, and you should demand your medical records if you're asking for them. Thanks.

[00:18:49] **Dr. Kyle Blackburn:** Yes.

[00:18:53] **Audience Member 1:** With regard to the medical records, I know most of the institutions that use My Chart, you can almost get them before your doctor does. You can download them. The problem is... that's a problem too. Sometimes as a therapist, it's like, why did I put that in the note? But there's a limit. It's like more than a year or so, it's not there. And so, then it becomes a problem. And then someone like me that was diagnosed 35 years ago; you're not going to find those records. So how do people who were diagnosed years ago fit into these study models?

[00:19:33] **Dr. Kyle Blackburn:** Well, that's exactly right. And I think that's where we're realizing those challenges. We may have put more challenges in front of us and we need to actually get good information. So that's where I think we're starting to walk some of these back and at least make some of these things optional because they would be nice to have on at least in a subset of people but starting to open it up to the community more and make this more inclusive. I think that's really my goal.

[00:20:05] **Audience Member 2:** Question from the--hi, Kyle--question from the online community came in. And the question is, are there any plans to extend the registry to include pediatrics?

[00:20:17] **Dr. Kyle Blackburn:** So, the registry does include pediatrics at the moment. So, if a child or their family member would like to enroll, they are able to do that. Thank you all.

[00:20:34] **Dr. Carlos A. Pardo:** Thank you, Kyle. For the record, Dr. Greenberg is not retiring.