

A Treatment Option for NMOSD

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[00:00:00] **Roberta Pesce:** Joining us now is Cara Amos, and our next talk is A Treatment Option for NMOSD. Please also take a moment to visit the Alexion AstraZeneca Rare Disease expo booth over the course of the symposium to learn more. But again, I'm joined by Cara Amos, Patient Education Manager at Alexion AstraZeneca Rare Disease. Cara, over to you.

[00:00:30] **Cara Amos:** Thanks, Roberta. Well, number one, it's amazing and wonderful to be part of this event. I look forward to coming together in person someday. But once again, my name is Cara Amos. I am actually the National Director for Patient Education Manager Team. So, I have a team of 10 that is waiting to chat with you in the booth. So, when you get a chance, go by, and see them. But today, we're going to talk a little bit about putting your health first in NMOSD. So, I'm not your Patient Education Manager, but I have a team of Patient Education Managers. There's 10 of them throughout the country, and they're here to help you along your journey, as well as your caregivers and your family. What do we offer? We offer education. We are going to talk a little bit today about an FDA-approved treatment option for NMOSD, and we're going to talk about how you can find support within your community as well.

[00:01:23] So, what are we going to chat about today? So today, we're going to talk a little bit first about anti-AQP4 antibody-positive NMOSD. We're going to review some of the disease basics, the role of complement, and relapses. Then, we're going to jump in to learn a little bit more about an FDA-approved treatment option for this disease. We're going to review complement inhibition, efficacy and safety, vaccinations, and dosing. And last but not least, and I think one of the most important things that I'm going to review today, is some of the helpful resources that we offer at Alexion to help you along your journey. So, let's jump right into the first section. So, let's talk a little bit about NMOSD, which many of you are very familiar with. So, a lot of this will be a review. But we know that with NMOSD, you just never know when your next relapse is going to occur. It's very unpredictable.

[00:02:19] So when you're living with NMOSD, the first thing we know is this is an immune disorder. It's a rare autoimmune disease that causes your immune system to attack your central nervous system, so affecting your brain, your spinal cord, your optic nerves. And we know that potential damage comes in the form of relapses. And we know that any NMOSD relapse can potentially cause permanent damage. There's uncertainty, relapses are unpredictable. They may be severe and recurrent, and each one that comes can

cause permanent damage. So, let's talk a little bit about the numbers and the risks. So, people with anti-AQP4, antibody-positive NMOSD, face a high risk of relapse, which can be potentially severe and recurrent.

[00:03:04] So I'm going to give you some numbers now. So, 73 percent of people with NMOSD test positive for anti-AQP4 antibodies. And, up to 93 percent of people with anti-AQP4 antibody-positive NMOSD have relapsed. So, I'm going to jump a little bit into an analogy to talk about the complement system, which is something that many of you may have heard of. So, what is the complement system? And why do anti-AQP4 antibodies activate it? So, the complement system's actually part of your normal, functioning immune system.

[00:03:40] And the way that I want you to think about this is that I want to compare it to a sprinkler system. So, the complement system is kind of like your body's sprinkler system. So, when complement detects a virus, a bacteria, a cold, it's going to go and eliminate them. So similar to how when a sprinkler detects a fire, it's going to turn on and try to get the fire out. So, when I look to the right here of the screen and we see that, the sprinkler on the right, so having NMOSD is like having a sprinkler system that's turned against you. When you have the disease, complement isn't doing its normal job. The sprinkler is just set off all the time, even when it's not supposed to be. So, it causes damage from all the water. You're getting flooding, you're getting damage.

[00:04:25] So really, as a follow-up, your doctor can help you understand, or your health care practitioner can help you understand the role of complement in anti-AQP4 antibody-positive NMOSD. We just gave you some conversation starters potentially to write down for your next visit. You can ask about your anti-AQP4 antibody status. You can talk about your relapses; no symptom is ever too small to mention or making sure that you take note of that so you can take it to your next appointment. And then risk. Focus on how you can reduce the risk of relapse, because we know that's an important goal to control NMOSD.

[00:05:04] So we're going to jump into the treatment section now. So, we're going to learn about an NMOSD treatment option called SOLIRIS. So SOLIRIS is the first and only complement inhibitor approved by the FDA for adults with anti-AQP4 antibody-positive NMOSD. So, what is SOLIRIS? I'm going to read this to you. So SOLIRIS is a prescription medicine used to treat adult patients with a disease, NMOSD, who are anti-AQP4 antibody-positive. It's not known if SOLIRIS is safe and effective in children with NMOSD. So of course, we always want to review the safety data and information, so I'm going to go through this with you. And as we move through the slide deck, you're going to notice that there's callout to the safety data on subsequent slides. But I'm going to review the next two slides in detail. So, important safety information.

[00:05:57] So what's most important that you should know about SOLIRIS? So, it's a medicine that affects your immune system. It can lower the ability of your immune system to fight off infection. So, it can increase your chance of getting serious and life-threatening meningococcal infections that may quickly become life-threatening and cause death if not recognized and treated early, which is why we're telling you about this now. So, number one, you must receive meningococcal vaccine at least 2 weeks before your first dose of SOLIRIS if you're not vaccinated. If your doctor decided that urgent treatment with SOLIRIS is needed, if you need it right then, you should receive meningococcal vaccine as soon as possible. If you've not been vaccinated and SOLIRIS therapy must be initiated immediately, you should also receive two weeks of antibiotics with your vaccinations. And, you may have had a meningococcal vaccine in the past, but you may need an additional one, and that's what you'll talk to your doctor about. So meningococcal vaccines, they do reduce your risk, but they can't prevent all of meningococcal infections, so it's important to recognize what are the signs and symptoms if you're on SOLIRIS of meningococcal infections. So, some of those are headache, stiff neck, fever, back pain, confusion, muscle aches and so on.

[00:07:10] So we actually give the doctor's office and your nurse a patient safety card that they'll be able to provide to you. And it's something that you'll actually be able to put in your wallet and carry with you so if you go to an emergency room or another doctor, they can be aware that you're on SOLIRIS. SOLIRIS is actually only available through a REMS program. And so, your doctor is informed about the patient safety card, they're informed about the fact that you need to be vaccinated. So, they will have all that information and sign off on that before they're able to prescribe you SOLIRIS. So, it's also important to know it may increase the risk of other types of serious infections, such as gonorrhea and certain fungal infections, such as Aspergillus.

[00:07:58] So back to the sprinkler system. So SOLIRIS is a complement inhibitor. There's that word, complement, again. So SOLIRIS inhibits complement, but of course the exact manner that SOLIRIS works as a treatment for NMOSD is not fully known. So, you can think of SOLIRIS as coming in and stopping that sprinkler from continuing to set off when it's not supposed to. SOLIRIS, once again, is the only FDA-approved complement inhibitor for the treatment of anti-AQP4 antibody-positive NMOSD in adult patients.

[00:08:30] So we're going to jump into the study and review a little bit of the study for SOLIRIS for anti-AQP4 clinical trial, it's called PREVENT, studied the safety and efficacy of SOLIRIS compared to placebo. It tested 143 adults with anti-AQP4 antibody-positive NMOSD, and the primary goal of the study was to determine time to first adjudicated relapse. What's an adjudicated relapse? We've given you a reference here. So essentially, it's an independent committee of medical experts that confirm that a relapse actually occurred in the study. So, here's the details of participants that were in the PREVENT trial. So, 143 adults anti-AQP4 antibody-positive NMOSD enrolled. 96 received SOLIRIS and 47 received placebo. The demographics are listed here for you. As far as IST use, immunosuppressive therapy, some patients were on corticosteroids or others, although some people only receive SOLIRIS in the study. Many ISTs, such as rituximab, were not allowed.

[00:09:34] What was the primary goal? The primary goal was to determine the time to that first adjudicated relapse, and the average number of relapses these patients had prior to enrollment was about two per year in the 2 years prior. So, the primary goal of the PREVENT study was achieved. So, I'm going to review a couple of numbers on the next two slides. There was a 94 percent reduction in risk of relapse for patients treated with SOLIRIS versus placebo. The primary goal of the PREVENT study, once again, was achieved and another number is 98 percent. In addition, at 48 weeks, 98 percent of patients treated with SOLIRIS were relapse-free versus the 63 percent that were treated with placebo.

[00:10:23] So once again, it's always important to review our safety information here and side effects. So, some of the common side effects, which we're going to see again on a subsequent slide, are listed here for you. Common cold, pain or swelling of your nose and throat, diarrhea, back pain, dizziness, flu-like symptoms, joint pain, potential throat irritation, and bruising. This could be a list of some of the side effects, not all of them, so we always want you to report back to your doctor if you're on SOLIRIS, any side effects that you are noticing.

[00:10:55] So once again, we talked a lot about meningococcal vaccine and getting it. And I'm going to talk a little bit about why that's important. So, you must receive that meningococcal vaccine at least 2 weeks before your first dose of SOLIRIS if you've not already had them. And, they're needed because SOLIRIS is that complement inhibitor, where we talked about the sprinkler system. And because your complement system normally helps fight off those infections, it increases your chance of getting meningococcal infections. So, we talked about some of the other infections it could also increase your risk for. And, we also talked about how getting these vaccines are going to reduce that risk. So, where can you get the vaccines? I think it's also helpful for us to share where you might be able to get those vaccines. So, your doctor's office, potentially at

a pharmacy, maybe a local health center or health department, and also travel clinics. But of course, your doctor or your health care team is going to be able to direct you on where to receive those vaccinations. And they will always refer to the most up to date ACIP recommendations.

[00:11:59] So how often do you receive SOLIRIS? It's an infusion. It goes directly into your vein via most likely a nurse. And, the dosing will occur in two phases. For the first 5 weeks, you'll receive SOLIRIS once a week. And then after that, you only need to get an infusion once every 2 weeks. SOLIRIS was studied as an ongoing therapy, so your doctor will discuss the treatment timeline with you. And if you miss a dose, you need to let your prescriber know. So, a lot of people want to know how long does it take? How long is the infusion? You can expect the infusion to last about 35 minutes, and then you can expect probably an hour of monitoring by health care professional, just to make sure that there's no infusion reactions, just to keep you extra safe. And, there's a point here about home infusion, which is a great option for some patients. But it's largely determined on your insurance.

[00:13:01] So continuing the safety information, we reviewed some of the side effects, but they're listed for you again here. And it's very important that any of these or side effects that bother you, that you report those. And there's information here on where you can report those. So last but not least, once again, we're going to move through a section of resources to consider in your NMOSD journey. So, OneSource, so you might ask what is OneSource? So, OneSource is a free and personalized patient support program offered by Alexion. So, my team of patient education managers, there's 10 throughout the country, we work underneath OneSource, and we are here to help you, along with the rest of our OneSource team. We can provide disease information to you and your caregivers. We can help with health insurance navigation. Help with ongoing support. So, if there's a life event where you need to change where you're going to be receiving your infusion or your insurance changes, OneSource is here to help.

[00:14:05] And then lastly here, community connections. I think that's one of the most important points here is that there's, we do offer in-person and online meetings and events. We're doing a lot more patient support meetings with my team. We can help connect you to advocacy groups in your community. And, really, we can help, we give you the opportunity to connect with others like you. And I'm going to talk a little bit more about that program next. So, I don't know if you've heard about Peer Connects, but this is a very exciting program that Alexion offers. So, we have a Peer Connects program, and we know that every person living with NMOSD has their own story to tell and own story to share. So now there's an easy way to talk one-on-one with another adult living with anti-AQP4 antibody-positive NMOSD. So, Alexion Peer Connects can help you understand NMOSD, its impact and how to live with it. Can offer their own personal story and journey and provide an understanding voice and support for you. And I'll leave this up just for a second so you can potentially jot down the website, NMOSDPeerConnects.com, or the phone number, where you can dial in and get connected with a peer.

[00:15:21] So, this is Glenice. Glenice, at the bottom, her picture, she is one of our stars, and she is somebody you could potentially connect with, with Peer Connects. So, she's there to connect with you. And this is a quote from Glenice. "Peer Connects is something I do because I really understand how it feels to live with anti-AQP4 antibody-positive NMOSD. When I talk with someone on the phone, they can bring up issues and I can share my point of view. Sometimes, I just listen. Other times, I can offer support or insight." I think for any of us, giving priority to your health and well-being is so important, even beyond NMOSD.

[00:16:03] So, we really want to call to action talking with doctor about your physical health, your emotional health, lifestyle and wellness, and potentially, if it's something that interests you, getting in contact with a support group. So really, it's your journey. It's your way. We are just really here at Alexion, here to support you along your journey. We want to be able to connect you with those in the community, potentially have you

part of our Peer Connects program, if that's something that interests you. We want you to stay connected, potentially with us through OneSource. And here at the end, I've got the website SOLIRISNMOSD.com. My PEMs are going to be in the booth all weekend, and they can actually direct you on how to find your own PEM in your area at AlexionOneSource.com, and we have a PEM finder there, where you can connect with your PEM. It's been a pleasure to be here today. Thank you for having me. And that's it.

[00:17:01] **Roberta Pesce:** Yes, thank you. Thank you so much, Cara. We appreciate you being here with us today. All right, I think we have reached the end of our stage talks for now. Please head to the sessions area to continue with your track talks. We're having some technical issues with one of the sessions, with L.A. Campbell. We are trying to work on it, so please stay tuned, and see you soon. Thanks, all.