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Complement Mediated Kidney Disease Series - A Patient and Caregiver Perspective

Announcer:
Welcome to KDIGO Conversations in Nephrology. This episode in our complement mediated kidney disease series titled, A Patient and Caregiver Perspective, is provided by KDIGO and supported by Apellis and Sobi. Here's your host, Dr. Carla Nester.

Dr. Carla Nester:
Hello and welcome to KDIGO Conversations in Nephrology. My name is Carla Nester. I'm the professor of internal Medicine and Pediatrics and director of the Division of Pediatric Nephrology at Stead Family Children's Hospital. And joining me to discuss a patient and caregiver perspective is Ms. Marianne Silkjaer Nielsen. Ms. Nielsen is the mother of a girl with who lives with immune complex, MPGN, and she's the founder of CompCure which is a not-for-profit association dedicated to improving outcomes in patients with C3G and immune complex MPGN. Ms. Nielsen, welcome to the program.

Marianne-Silkjaer-Nielsen:
Thank you very much for the opportunity to be here and support you in shining the light on complement mediated kidney diseases.

Dr. Carla Nester:
We're going to begin our discussion today with a few questions and we'll give you plenty of time to answer. What is the most difficult thing about being a rare kidney disease patient or parent, or particularly for C3G or immune complex MPGN?

Marianne-Silkjaer-Nielsen:
Although it is difficult to generalize, also, keeping in mind that there are more than 300 rare kidney diseases, of which many are heterogeneous. I think that most of the affected people share the following challenges, which also hold true for C3G and IC-MPGN. The first challenge is the diagnostic journey. It's typically long. We all know that it takes five years on average to be diagnosed in rare diseases. The diagnostic journey in C3G and IC-MPGN can also be very long. It is a challenge that kidney diseases can be silent killers with unspecific symptoms such as fatigue and hypertension. This could be so many different things, and that makes it difficult for the doctors, but also for the patients. Diagnosing C3G and IC-MPGN requires a pathologist who knows what to look for, and they're not always so easy to come across. Some patients in our community are lucky to be diagnosed early through screening programs, but most of the people in our community experience severe symptoms by the time the disease is detected. Many were hospitalized. This was also the case for my daughter, and she needed two biopsies to get her exact diagnosis. I think it's important that we remember that the diagnostic journey is difficult to navigate even for resourceful families living in rich countries. As a result, I believe that many patients are not being diagnosed at all.

The second challenge is that most rare diseases are often facing a serious progressive condition and realizing that there's no cure or disease-modifying therapy is frightening and devastating. Life never becomes the same again. You will need to deal with a new level of stress and anxiety while managing the disease.

The third challenge is related to the heterogeneity and insufficient disease understanding. The likelihood that your treating physician has widespread experience in your specific rare disease is slim. It takes time, courage and commitment to find experts, which is very important. Data privacy and territorial approaches to data sharing pose several challenges for building proper levels of long-term

evidence needed to inform clinical decision making and guidelines. As a result, many patients perceive the clinical practice as experimental and disproportionately influenced by short-term financial priorities. Which can lead to suboptimal levels of care, poor outcomes, and even premature death.

Dr. Carla Nester:
Thank you, Marianne. That's, that's very insightful. It's an important set of challenges you've reminded us that exist. If I asked you, is there one in your mind that's a priority or are they all equally important?

Marianne-Silkjaer-Nielsen:
Hmm, that's a difficult question. I mean, I think they're connected. So in order to get the right level of care, you need to get it timely, and that requires that you're diagnosed timely. And you need to have the nephrologist taking care of you if they have the right level of experience. It's just easier for them to make the right decisions. And then they know what to do, and, and that's not, you know, always the situation that, that makes it very, very difficult for, for the treating physicians in, in rare diseases in general.

Dr. Carla Nester:
Thank you. Through your child's journey or through your journey, through this process, were there steps where you thought to yourself, uh, you know, wow, this could have really gone differently or better? And, and if so, do you have a suggestion for how it could have gone different or better?

Marianne-Silkjaer-Nielsen:
It's a good question, and before I answer it, I just wanna emphasize that all the physicians involved in treating Esther, my daughter, they were extremely skilled and they just did an amazing job. But nevertheless, she ended up in the intensive care unit in coma with sepsis and a mild stroke. So we spent two months in hospital, primarily in a room with five other families. There was this constant level of noise and anxiety, and it was just impossible to really shake off. So I believe that the long hospital stay and the severe complications could have been avoided if Esther would have been diagnosed earlier. Hence, I think that screening programs something that should be explored. I mean, not specifically to C3G and IC-MPGN, but for kidney diseases in general. And being diagnosed with a kidney disease is not enough. It's really, really important to know what kind of kidney disease you have. So that is the first.

The next one is related to the therapy. So Esther was treated with steroids, pulse therapy three times. We did not really see any effect, but the side effects were terrible. I honestly couldn't recognize my daughter physically and psychologically. When her disease got really serious, she was initiated on a targeted therapy off-label, and it worked. Nevertheless, we had to fight pretty hard to get access to this medicine. So I think that access to innovation before critical illness and while there's still kidney function left to preserve is really important. And also here, I think that experts and strong guidelines are of critical importance.

We need to keep in mind that critical illness and long hospital stays impact the entire family. The siblings are neglected. In our case, Esther's twin brothers who were not even two years old, didn't really see me for two months. I remember that I feared that they would forget about me. My husband and I took turns staying in hospital with Esther and going to work every second day. During the night, I would always stay in the hospital with Esther. Both of our employers went above and beyond to support us. And I think this is really rare. When going through a disease journey like this one, employment is mostly impacted. Which should also be consistently considered in health economic discussions.

Dr. Carla Nester:
Thank you for sharing your story. Unfortunately, I suspect as you've indicated that there are others that have gone through similar presenting stories. I also see your point about the advantage of early diagnosis. But what I also hear from what you are saying is, is that physicians need to better understand or at least recognize just how traumatic some of these early events can be, not only to the patients, but to the family unit. I think it's an amazing thing for us to just keep in the back of our minds going forward. When thinking about the next decade of care for C3G and immune complex MPGN, what do you think will be the most impactful?

Marianne-Silkjaer-Nielsen:
I think there will be much more knowledge about the diseases and more options on how to treat them. I also think that there will be more focus on early diagnosis. The outlook today is completely different than it was six years ago when my daughter was diagnosed. This is extremely positive.

Dr. Carla Nester:
I agree. I'm curious, and I don't want to put you on the spot, but how do you think we should spread the knowledge better?

Marianne-Silkjaer-Nielsen:
I think it's important that we think broadly here and that we communicate outside, you could say, of the normal bubble. So that is also to primary care physicians because they're the first ones typically seeing these patients.

But I actually also think we need to communicate, with a normal population and leverage whatever channel we can. So I can say for our little association CompCure, we leveraged a celebrity. We were in national television, both in Germany and in Turkey. We had the opportunity to present in the European Parliament at a site meeting of the World Health Assembly. And this year we actually reached more than 25 million people.

Of course, we don't only talk about rare kidney diseases or these, you know, very rare diseases. IC-MPGN , and C3G. We talk about kidney diseases in general, but we don't forget to mention how important it is to get the exact diagnosis. 40% of people in dialysis in Europe, they don't know what disease they have and probably many of them have a rare kidney disease. And it's just extremely important that they are being diagnosed correctly, because that's a prerequisite for getting a targeted and optimal therapy,

Dr. Carla Nester:
Of course. Sure. So, are there things that you're worried about going forward?

Marianne-Silkjaer-Nielsen:
I mean, I'm less worried now than I was six years ago, but I must admit that I still worry a lot about the future. The long-term reality is that we are fighting a battle against oblique prognosis. I do however, believe that we have a chance to win this battle, especially if we work together as a community caring for each other, sharing our expertise and our data with a common objective of helping patients to get diagnosed early and treated optimally. This is the mindset we have in the patient community, and it gives us a lot of hope and encouragement.

Dr. Carla Nester:
If you're just tuning in, you're listening to the KDIGO Podcast on a patient and caregiver perspective. My name is Carla Nester and I'm speaking with Ms. Marianne Silkjaer-Nielsen. So, as I'm listening to you speak, I'm thinking to myself, do you have a concept of whether the major challenges will be the same in the next five years as they were for those that you faced previously?

Marianne-Silkjaer-Nielsen:
Yeah. I think that the challenges we just talked about could still be there in five years from now. Furthermore, the current geopolitical environment and macro trends pose additional challenges to the global health systems. Rare diseases could end up being further deprioritized. I think these could be additional arguments in favor of doing everything possible to avoid critical illness.

My dream is that all patients living with C3G and IC-MPGN will get access to the right level of care in the future, personalized to the individual needs. On this premise CompCure was founded. Through broad global collaboration and partnership, CompCure is now active in more than 20 countries, and our global growing registry includes data for more than 300 patients. We are in the process of getting email regulatory grace data certification, and I also hope that we will be able to support the development of guidelines in the future.

To conclude, I believe that together we can effectively address the challenges we are facing while supporting all patients in being diagnosed early, treated timely and optimally, and getting a chance to live healthy and fulfilling lives.

Dr. Carla Nester:
Before we close, are there any final messages you would like to leave with our listeners?

Marianne-Silkjaer-Nielsen:
I hope that this podcast can inspire awareness and reflection on the broad impact C3G and IC-MPGN can have on the lives of patients and their families. If you would like to learn more about CompCure and our activities with focus on C3G and IC-MPGN, we will look forward to hearing from you.

Dr. Carla Nester:
I want to thank my guest, Ms. Marianne Nielsen for joining me. It was a great pleasure having you on the podcast today.

Marianne-Silkjaer-Nielsen:
It was an honor for me to be here. Thank you so much for the opportunity and for your incredible professional and personal commitment to support our community.

Dr. Carla Nester:
My name is Carla Nester, and I'm a rare kidney disease specialist. To access this and other episodes in our series, visit kdigo.org/podcast.