**Transcript**

**Advocating for Rare Patients with Wendy Erler**

Stan

Hello everyone and welcome to the n-Lorem podcast series. Today, we're very pleased to have a special guest. Wendy Erler will be joining us from Alexion. Wendy is vice president and head of patient advocacy at Alexion Pharmaceuticals. She has an entire careers worth of history in trying to make sure that the patient’s voice is a central driving force in what we do in our industry. I think her experience will be highly instructive in a number of ways.

Wendy

Thank you so much, Stan. I'm excited to be here.

Stan

Well, it's great to have you. So, I know a bit about your work history, but I don't know anything at all really about the rest of your days. So why don't you tell us a little bit about how you came to be where you are and all that.

Wendy

Well, on the personal front, I had a really lucky upbringing in that I grew up with a father in the military, so he was career Air Force, and a fighter pilot, which as a kid, it's pretty exciting to be on the flight line and be up close and personal to that. But that meant that we moved a lot. So, I've lived in many, many different places across the US and a few places outside of this country. But I think that part of my experience is so relevant to what I do today because I've spent my whole life being the new kid in town and connecting with new people and getting to know new communities. And it's always been really important to me to really join a community versus come in and try to change anything. So, grew up in the Air Force, went to College in Ohio, and pretty quickly out of college decided I wanted to be in pharmaceuticals and the industry with one sheer personal purpose of really serving patients in any way that I could.

Stan

Well, it's an interesting history. So, did your dad happen to fly the F-35 or anything like that? Or maybe that's a little, he's no longer on the flight line, I imagine.

Wendy

Not now, but we were in the F-111, he flew F-15s, we were in A-7s for a while, so a lot of different aircraft. And to quote my dad, "each and every one of them has its own personality."

Stan

I'm sure that's true. Now, that would have been nifty. Maybe I can hit you up to get in on one of those planes one of these days. That'd be really cool. I know you've been interested in the patient’s voice but why don't you walk us through some of your early experiences in the industry and how those experiences you know, shaped to an even greater degree, your interest in trying to make sure that we focus on the patients in our industry.

Wendy

Thanks so much for asking. And I was really lucky to join our industry at a time where I think drug development was just really experiencing quite a renaissance of science advancing and understanding what patients needed. I started my career at Glaxo, and it was a small company at the time, and we had a lot of innovation. During my tenure there, we launched very common medicines today, but things like Zantac, Zofran and oncology which treated pre chemotherapy, emesis and hyperemesis and pregnancy. So, a drug like that actually enabled patients to then be treated for cancer because they could tolerate the medicine, so innovation happens all around us. I then was really lucky to land in oncology at a time where research and scientific understanding of oncology was just such at the forefront of science, but more importantly, how patients were able to tolerate therapy, how patients were able to continue to work while they were on therapy and the surround sound of experiencing life with the condition that could be treated. And kind of, you know, started to put my finger on some of the company decisions that were being made, not just with my company but others seemed to be very focused on getting our messages out and our products out and less focused on what do patients and caregivers really think is important. And so, when that idea started to crystallize in my head, I looked to leaders within my organization to find ways to actually think about how do we bring the patient voice in. And that's a concept today that's talked about a lot, but at this time you know, in the early 90s, I wouldn't say it was talked about as much. And then something happened externally that really brought this to life in a dramatic way, and one was the HIV movement, and the other was pink ribbons and breast cancer. And suddenly, patient communities seemed to be really activated and had a place where they could voice what they needed and what was important, whether it was legislation, or drug development. And that all clicked for me, that I had an opportunity to help bring those voices in and really find roles and find organizations where that mattered and could impact and drive decision making.

Stan

Yeah, you know, I think people don't really understand how important the HIV patient movement was in establishing patient advocacy and I think changing attitudes across the industry and at the regulatory agencies as well. And as a result, I think we've seen just tremendous growth in the power of the patient groups to influence decisions both in companies, but also in the regulatory agencies, not just in the US but around the world.

Wendy

And influence for all the right reasons, right? And if we think about the impact of having a devastating diagnosis in a family and then what happens next, our patients and caregivers are really the only people in this ecosystem that didn't choose to be here. They didn't go to medical school typically, they're not trained, they're not being paid to be in positions to be involved in this, it's a diagnosis. It's the most critical time of their lives, and then all of a sudden, you're thrust into this role of patient or caregiver. So, we have to give them the platform and the opportunity to have their voices be heard.

Stan

You bet. And so, at Glaxo, of course, it was almost entirely in those days small molecules. And then I think you went off to IDEC. So now you're in monoclonal antibodies. And from there, at some point you end up at Wave. So now you're in, you know, ASO therapy. So, I don't know whether you planned it, but you've now had experience in all the major platforms for drug discovery, which is kind of cool.

Wendy

And such a privilege, right? But I think the other thing that happened during that trajectory was that I was really, really lucky to begin to work in rare diseases. And IDEC merged with Biogen. So, we became Biogen IDEC and most of my career up to that point had been in oncology and working with oncology patients, and Biogen had this kind of unknown pipeline in rare diseases. And I was so lucky to get to work on an asset for ALS and really get involved in the ALS community and that's a community that, you know the diagnosis is uniformly fatal. There are no meaningful treatments that are really changing life expectancy. And that community had very clear directives on how they wanted to be involved in trial development, what they expected from therapies, and very vocally against placebo, for example. So really had to engage with the community and work with them and be able to bring a clinical trial program forward. We also had a partnership with Ionis for Nusinersen. And again, I literally, Stan, one morning was sitting in my office and a woman called from California, and it was 8:00 AM East Coast time so 5:00 AM her time, and she wanted to know why our clinical trial had an age limit for newborn babies, and her baby missed the cut off by two weeks. Two weeks. And that then sent me off on a trajectory of really wanting to learn and understand the ethics around trial design and frameworks of things like enrollment criteria. How could you say no to somebody whose baby was not eligible and then definitely not going to live from a disease like SMA but within the ethical constraints of trial design and what we were trying to do for the broader good. And as a result of that experience, when I got to Alexion, I actually set up an ethics advisory board. So, we have five external ethics advisors that we engage about once a month on a wide variety of topics.

Stan

I think that's really important innovation. As you know well, I was responsible for SPINRAZA, and consider the decision I made to treat the first group of patients, probably the scariest decision I've made in my career, spent many, many hours talking with parents who were in similar situations and always regretted that there were really no sensible solutions to get these babies treated earlier, and particularly as we look back now, we know that if we are able to treat SMA infants before they become symptomatic, the majority of them grow up like normal, healthy children, always had my heart broken about the patients, the parents and so on, who just missed that opportunity and the answer to that in the end is to do more research and get the drugs made earlier and better. But it was a grand journey, yeah.

Wendy

Stan, you said that word fear. And there's a huge responsibility we have in drug development and particularly rare disease with families. It's so personal and we know these families. But it is, drug development is hard, and it's scientific and it has to be rigorous. And so, there's also an opportunity for us to just be as transparent as possible with patients and participants in the clinical research process so they can better understand how we and why we make the decisions we do.

Stan

You bet. I think it's not understood at all by the general public that we're really the only industry that's ever existed that as a part of our day-to-day life, have to make decisions that could affect the lives of other human beings and do. And that concern about the potential of doing harm has an extraordinarily powerful influence on everyone in our industry, and increases the emotional stakes, and then to a level that I don't think you can really appreciate until you've really experienced it. And so, you also then spent a few years at Wave, a newer antisense company, that I think you know I take justifiable pride in thinking that the space exists because of the work that we did at Ionis. So that gets you into the third platform and the platform that we are using at n-Lorem. I'm interested in what you think you learned out of your experience in the three platforms and some very different companies. Wave was a much smaller, newer company than Biogen or IDEC or certainly Glaxo.

Wendy

Yeah, so really good question, and Wave and the opportunity I was presented with at Wave are really a height of my career. But personally, I'll share with you why I made the decision. I had spent 14 years at Biogen IDEC, and literally loved what I was doing, and working in some very important disease areas and working with, you know, mentors like Al Sandrock and you know big, big, scientific experts. But I had an opportunity to interview at Wave and the CEO and the SVP of Business Development said to me, and this is really relevant for this conversation, "we have this very exciting platform technology called antisense oligonucleotides." We add a stereo pure approach, so a little bit of a unique difference in the way we were doing the chemistry, but what they said to me, Stan, is "we want to get to a place where we can serve n of 1 rare diseased patients. And I just, my world exploded in. Can you imagine if we have a technology that's flexible enough that we could serve n of 1, you know, hundreds of patients, but one patient at a time? I wanted to be a part of that, to the point where I left that first interview and I sat in the parking lot, and I called my husband and I said "I don't care what this offer looks like. I want to work with these people." And so, I took the leap, and to answer your question, I think there's something really special about antisense and I was so lucky to learn about that approach because you can do a lot of manipulation in the lab where you're not implicating patient tissues, and patient blood, and doing these things that really, you can do a lot of the experimentation in the lab and get a pretty clear direction of travel, so that you have an understood kind of safety profile, maximum dosing and some of those really important questions largely confidently figured out before you go into people, and we really had an aggressive approach to accelerate going right to phase two phase three, versus this long decade plus trajectory of drug development. And that really excited me because no one in rare disease has time to go through the typical drug development process.

Stan

Let's move on to n-Lorem, which is a nice segway, thank you. And Alexion, late last year, early this year, gave us an extremely generous donation. And I know you were instrumental in that. Why don't you tell us about how that came about and why you think that's so important for Alexion to be doing at this stage.

Wendy

So, I'll start a little bit at the beginning of my career at Alexion. I joined the organization over three years ago. And really came to Alexion because of the opportunity to, in a meaningful and tangible way, not just embed patient centricity, but build frameworks and organizations so that we could change behaviors internally and really organize and deliver on making decisions around patient needs. So, there was a very big opportunity from a leadership perspective to design these frameworks and build the organization to do that. And to me, that was game changing. This was a company that wanted to, in a legitimate, measurable way, say we want to be patient centric. Here's what that means. Here's what that looks like. Here's how we're going to invest to do that. We are a rare disease company, and we work in ultra rare diseases as well as kind of bigger rare diseases. And Alexion is celebrating its 30th anniversary this year, but there was a keen opportunity to really be clear about what we mean around patient centricity. So, I started, as I said about three years ago, I've built an organization with capability and patient advocacy as well as leadership in organizing around global patient insights to drive decision making. We have some very unique and special programs that we've developed. One is called STAR, Solutions to Accelerate Results for patients. That's an innovation accelerator where we bring in all of our cross functional colleagues and external patients, stakeholders, physicians, payers etcetera and breakdown silos. And just in a really design thinking way, how do we choose those moments that matter where Alexian can have an impact in patients’ lives? And develop solutions quickly to serve those moments that matter. And then we have another program called LEAP that is a simulation program where we have our employees go through a simulated experience of what it's like to be a patient and walk in their shoes, receive a certain diagnosis, and then start on that journey. We have a program in development right now for LEAP that is focused on what's the clinical Trial experience like? It's not really about blood draws on the day of the clinical trial visit, it's everything that it takes a family to get ready to go to that clinical trial, visit or consent for a study. So, this LEAP experience is to have our employees understand what are we really asking from patients and families if they join a clinical trial. All of that being said, I was in the right organization at the right time that when I had a chance to get to learn about n-Lorem, we as a company at Alexion, we are committed to supporting patients and their families affected by rare diseases, full stop, not just our rare diseases, but rare diseases. So, it was really important to us to make this donation and to continue to support n-Lorem's development of individualized medicines for one nano rare patient at a time. And why are rare diseases important? There are so many reasons. There are so many unsung heroes in rare disease. But fundamentally, research on rare diseases is really important because it can lead to the discovery of other things we need to learn and other components that may be affecting common diseases and then aid in developing even more effective medicines for other conditions. But I also really believe personalized medicine is the future, and that's what n-Lorem fundamentally is going to deliver, personalized medicine.

Stan

And so, you went to your CEO and said, I've got a deal for you, you can give some money away to these crazy people pioneering this nonprofit model?

Wendy

You know, I was really fortunate to have an organization where I felt that I could do almost just that, Stan. So, I did. I talked to our CEO and I, you know, really, your story is amazing. What n-Lorem wants to do is incredible. And I think all of us should be a part of it that can. And so, I was able to have a conversation around, we've agreed as an organization that our commitment is to rare diseases. We want to be a leader in rare diseases. And then what does that look like? If it's solely focused on advancing our medicines, then that is still very self-serving and not as patient centric as we want to be. And he was completely aligned with this is work, the work that n-Lorem is doing is work that matters and is important for rare disease patients. And if this was a way for us to be a part of it, he was fully supportive.

Stan

Well, that's wonderful. Well, I look forward to getting to visit him. I've known Mark for a bit in his various iterations of his career, so I'm sure we'll get to that one of these days. As I described in our presentations n-Lorem industrializing this process, and I think that's critical because industrializing means that you assure the highest quality at each step, and that you're capable of expanding to meet the needs of not just a few hundred or one, or but many, many thousands, to actually millions. And for me, n-Lorem has been very much like returning to the practice of medicine. It's that intimate, one patient, one family experience at a time, that is rewarding in a very different way from you know what I've done in my career, in the industry, and you're certainly right that there's an incredible amount of knowledge to be gained in what we're doing. This is a unique enterprise. I do think of it as a core of discovery both for the mind and it's certainly for me the core of discovery for the heart. Every day is a core of discovery for the heart for me. With your experience I think we're going to lean on it a lot in the coming years as we develop our podcast series as a part of the Patient Empowerment Program, I think every time I talk to you, I learn a little bit more about how I should do that and how we should do it better. And so, I'm looking forward to involving you and all that.

Wendy

And I'm honored to have the opportunity and you know, Stan, as I hear you speak, I think so much of this is fundamental to human interaction, right? We're really talking about a relationship building business. We're talking about listening and learning. I had a woman reach out to me a couple of years ago and I'm not sure how she got to me even, and her daughter was diagnosed with an ultra-rare disease and they, you know, went through that trajectory of shock and grief and confusion and very quickly got to the place of we need to understand this. We need to invest in research. You need to look at drug development. You know when you engage with people like that and so much of the responsibility sits on their shoulders how can we alleviate some of that? How can we break down some of those barriers? It's a whole new language and a whole new process of fundamentally trying to make connections in academic institutions or with regulators. And so, I think it's a little bit of a relationship business, and how can we help people make those connections, and breakdown those silos? And the other thing that I'm so impressed with n-Lorem and I'm interested to hear you talk about industrialization because the way I think about it too is we're organized to deliver the results we get and by that I mean if you don't have the infrastructure to work fast and to get answers and to, you know, figure out the compliant legal way to get things done, we're just going to continue to do things the way we always have. And you're building an organization that's knocking that all away and enabling this very rigorous research to move quickly.

Stan

Well, I couldn't agree with you more. And I've been so tremendously impressed with the people I've met who have no training of any sort in the sciences, not a bit, who managed to learn so much and ask the right question, which is always the test of whether you understand something. And I'm hoping that of both the interviews that we're doing and the podcast series and the sort of lectures that build from the basics on up will really help people shorten the time and energy they have to invest in learning how to think about this so they can focus on themselves if they're the patient or they can focus on their child if their child is the patient. And so, we're working at it and there's just not enough of us and not enough time every day to make the kind of differences that we'd like. But over time, I think we're going to do that.

Wendy

And you're building community and I think that there's a support network too, just in having a place for people to be heard. That's so important.

Stan

Yeah, I was stunned at the isolation of the nano rare patient. It just, there is no community if you're the only person who has a mutation. And so, I do think one of the functions that we're taking on at n-Lorem is that creation of a community of nano rare patients, even though they may not share the same mutation, they share the same challenges to life and to a family that comes with all this. Anything that I haven't asked that you'd like to say to nano rare folks who are listening?

Wendy

Just one last thing that I don't think we touched on, but it's really important is I think what compelled me to really do everything I can to support n-Lorem is the actual opportunity that antisense brings to the table. So, we talked about some of the other small molecule, monoclonal antibodies, but there's something really unique and special about antisense because of the regulatory pathways and again a lot of this is about you know changing the ways things have already been done in the past. You've set the stage to be able to do that with antisense and that's something else I'm really excited about.

Stan

As am I. It's one of the many reasons that I founded Ionis 30 years ago. I realized long ago that the industry was dying because small molecule drug discovery just isn't efficient enough. And you really can't learn much. It changes things. And with antisense we know the rules and we've really taken the technology from a blank piece of paper to where it is today, and it is unique, extraordinarily efficient and rapid and versatile. So, without technology, of course, there's no way we could be even thinking about this. And that again is this convergence of basic science in genomics and the work that we did in antisense coming together at this moment, when we're beginning to learn that the nano rare mutation and the nano rare patient is actually a vastly more common event than we realized even two or three years ago. We're glad to be in the forefront of that and driving the model.

Wendy

And the only thing you know, given all the world events and everything that's happening today n-Lorem is servicing the world and that's I think, really important too. These families are, they can be anywhere, it can be anybody in any race, in any country, and so really thinking about that responsibility for patients across the globe.

Stan

Well, it's an awesome responsibility that I've had the privilege of participating in and sharing for more than four decades, and this is the best possible conclusion to a career that I could imagine and one of the big pluses of it is I'm spending much more time meeting all the other people who share the heart of this endeavor. So, Wendy, thank you very much for joining us today. And more importantly, I thank you for all of your efforts on behalf of patients of all types and now for the patients that that we're focused on at n-Lorem. It's great to have you on board and we're going to be depending on you in a lot of different ways.

Wendy

Thanks so much, Stan.

Speaker 3

n-Lorem is a nonprofit committed to discovering and providing personalized experimental treatments for free, for life to patients with genetic diseases that affect 1 to 30 patients worldwide, referred to by n-Lorem as nano rare. Many of these patients progress and die without ever achieving a diagnosis. This is where n-Lorem comes in. They do the impossible by providing hope, and for those that they can help, free lifetime treatment. For more information about n-Lorem or today's episode, visit nlorem.org. Any questions can be sent into podcast at nlorem.org. Search n-Lorem on Twitter, Instagram, YouTube, LinkedIn and Facebook to connect with us. Please rate and review the podcast on Apple, Spotify, or wherever you listen. This truly helps us climb the charts and allows others to find the show. This podcast is hosted by Dr. Stan Crooke. Our videographer is Jon Magnusson of Mighty One Productions. Our producers are Jon Magnusson and Kira Dineen of DNA today. Thank you for listening.