May 8, 2019

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Taren: Jayne, welcome to the PharmaVOICE WoW podcast program and congratulations again on being named the PharmaVOICE 100 in 2018 and on your recent bio and J Award that we'll talk about in a little bit.

Jayne: Thank you very much Taren, I really appreciate the invitation to be here and looking forward to our conversation.

Taren: Me too, anybody who's gotten a chance to know you in the rare disease space knows that you are a legend in that rare disease community but for those who haven't had the pleasure and privilege to get to know you and hear your story, could you talk to us about what drew you to the rare disease space and then as a follow up you've played such a unique part as well becoming one of the first and still one of the few chief patient advocates in the industry, I want you talk about that part of your journey as well. Well, let's start about with what drew you to the rare disease space?

Jayne: Taren, it's really interesting what drew me to the rare disease space because it was not a space that I knew I was going to and I think as what happens in many people's career you follow your own development and your own interests and you seize opportunities and they sometimes take you to very unexpected but very special places and I would say that's what happened with me in rare diseases. My background is not in science. My background is not in medicine. My background is actually in journalism and as a journalist always very curious, always interested in learning new things and taking a picture to help other people understand what I've had the privilege to see and I moved from newspaper journalism into corporate communications and marketing communications and then took that into the nonprofit space. While I was working at a large healthcare and social service agency in the greater Boston area for many years I had the opportunity to start to talk to people about genetic education. In fact there were industry professionals who came to me as well as we had the opportunity to talk with some academics people in genetics, the leader of one of the country's first genetic counseling masters programs to find out how we could reach certain people in particular in the Jewish community and in the community of people who came from the former Soviet Union back in the 1980s, how could these people be reached to help them understand genetics and what their particular risks were for either being a carrier or

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developing a rare genetic disease. And so with marketing and education and community programming expertise we worked together to try to get some of these messages out and frankly it was the first time that I had a connection to genetic disease and genetic disease education.

From that work, I was introduced to the National Tay-Sachs & Allied Diseases Association, one of the country's oldest if not the oldest genetic disease patient advocacy organization. They were part of this group that was developing a genetics education program in Boston and they then approached me some many months later because the executive director was leaving and they asked me if I would be interested in applying for the position of Executive Director and the rationale for them at that time was they wanted someone with marketing, with good communication skills who could help them to bring their organization up to the next level and part of that was also fundraising and friend raising and I had done a lot of development work prior to that in other non-profits.

So it took me to a very interesting opportunity but as I said earlier one that I did not have a personal connection to and certainly I didn't have a training connection to. Certainly myself and my family had different medical experiences as many parents and their children do and our family seemed to have some unusual ones when I was a kid, so I always felt very comfortable in a medical environment talking to people, talking to physicians and felt that I would be able to make the difference that they were looking for but I really had to throw myself in to understand these diseases, to understand what was happening with research which was really still in its infancy back then but what was really happening with the families, the people who were getting diagnosed and in particular the children because most of these diseases affect very young children and what were their needs and what were they looking for in terms of support, support from some infrastructure, support from each other, and how are they going to get through a death sentence diagnosis. No qualms about that. These are death sentence diagnosis, terminal illnesses of children, neurodegenerative and very, very difficult with not a lot of research going on at that time.

And so for me it was the connection with these people as I said I was fortunate myself not to have a personal connection to one of these diseases but to be able to speak with them, to understand what they were going through as much as I could, work with my staff and work to make a difference and through the organization, was able to double the budget which was one of the remits that I came there with, and also expanded research in terms of funds that were amassed to support early research in these neurodegenerative diseases, this life of someone's of storage disorders, and also to gain more attention from other researchers about these diseases and that was something that we were able to accomplish.

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Taren: Your journey has been fascinating and to think that you started off from a newspaper journalistic background to now leading one of the most renowned rare disease companies in the country or in the world as the Chief Patient Advocate is really quite something and being involved with those families and having that exposure to such sadness and sorrow but I would also imagine to such possibility and innovation. Describe what that's like for you in your role?

Jayne: I think that that's you're right, it is sadness, it devastation, a possibility for sure but what I always got from families when I was working on the nonprofit side and certainly still here at Amicus is the strength and the resilience that you see these individuals, the parents, the children themselves what they have and how they look at their situation, devastation for sure. Grief is a continual grief that sort of comes and ebbs and flows when people think of what they had envisioned and then where they're at and certainly lost but it is that resilience and that strength and knowing that whatever you do it is appreciated because you're taking time to try to understand them and their experience.

I would say that that's where a lot of my communication training came into play. But also what I see in meeting many other patient advocates in industry that many people come from a communications or marketing communications background and that's because it's about connection to people. How you connect to someone, how you learn from them and they from you but also how you develop relationships. I really believe that that's the key and being authentic and being honest and being transparent in how you communicate.

So for me to have come from a very different background and I've had many different wonderful career opportunities and then to move from the non-profit side to the for-profit side was really an honor and I would say that having the ability to do that here at Amicus Therapeutics has been more than an honor because it's helping to frame the way the company has been interacting with the disease communities since its earliest days is this approach of certainly high regard and respect for people living with these diseases, trying to understand their experience, and thereby trying to make a difference. And we try to make a difference obviously in the development of innovative medicines but even in situations where we have been less than successful we're still able to make a difference and maintain those relationships with education, with resources that we develop that are independent if you will of the specific products that we may be developing. It's looking at what are the commonalities that people living with rare disease have, what is it that they need to know maybe about their disease but what do they need to know about the space that we're in.

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There's a very trendy term right now but the ecosystem that we're working in. And so they need to understand what a clinical trial is. Certainly a person with a diagnosis or a parent of a child with a diagnosis one of the first things they do is they want to say, "what will help us?" And they will do research and they'll reach out to disease organizations as they should and they'll go online even against sometimes a physician's advice and they want to see who's doing what in this disease, who might be able to help my child or help myself. And they want to know about clinical trials and they want to know about the research but they may not fully understand what the broader sense is in terms of what is a clinical trial, where does it fall in the whole drug development process, what is informed consent.

Sometimes there is truly a desperation and people just they want in, they want a chance to be saved and it's important that people understand what the full aspects of informed consent and how can we as advocates be it an industry or be it on the disease non-profit side, how can we help people to gain a deeper understanding of the whole process so that they truly can be our partners in it. And I think that's what's very important is educating people, helping them understand what it is they want to learn, helping them learn it, helping them become empowered so they can be a partner in their healthcare and in the drug development process.

Taren: Inspiring and fascinating it's all great stuff. You touched on a minute ago about innovation and I wanted to talk about some of the things that you have been personally responsible for in terms of innovation and one of those is the Amicus Therapeutics Patient Advisory Boards Program which was launched back in 2007 and my understanding is one of the very first of its kind and now it's almost an industry standard. You're also responsible for Our Good Stuff which was a patient inspired initiative. I'd love for you to talk to us about what those initiatives look like today and where you think they're going to go in the future.

Jayne: What's so exciting about the two things that you've mentioned, Patient Advisory Board Program and Our Good Stuff is that they seem to be anchoring us in many ways as far as our culture here and the culture that we share with people in the rare disease space. So to first address the Patient Advisory Board Program that you mentioned which yes did start back in 2007 and I'll just briefly give a little bit of context, the history is that back in 2006 and early 2007, we held a few different patients summits here at Amicus and our first of headquarters here in Cranbury, New Jersey and what we wanted to do because what we were developing at the time of pharmacological chaperones is a very different approach to thinking about life as some of storage diseases, what might cause these diseases, and how they might be treated. It was really a paradigm shift. So we wanted to be able to educate the patient community so they could understand what we were doing,

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so that in turn we could help them and develop the right kinds of clinical trial protocols that we could understand what was meaningful to them and build that into our clinical programs et cetera and we had a one day with each of these different disease communities at the time. We got into so much not only about what we were doing but more importantly what were they experiencing and this was in the days before the Affordable Care Act when people were trying to do whatever they could to maintain insurance when there were pre-existing conditions and for people with rare diseases that's just an Everest of a challenge and understanding what it was like for them to get a diagnosis, what was that honestly like for them, what was it like to communicate to physicians in many cases physicians who might not be expert in their particular rare disease but they're their healthcare partner.

So we had a lot of things that we discussed and one of the things in one particular summit it actually was our Pompe Patient Summit where we had both people living with the disease as well as some spouses and caregivers. We had a breakout session that was for the caregivers and we were asking them how are they doing and what were their needs and what could be done to help them as a caregiver of someone living with Pompe and in this case with late onset Pompe. Great conversation and people shared a lot but towards the end this one woman turned and she said, "you know we've been able to participate in similar kinds of meetings with other members of industry and we certainly appreciated that opportunity" but no one has ever directed the conversation to us. It's always been directed to the person, the individual with the diagnosis and they were just so appreciative that we wanted to know what would help them. And it was at that point that I realized that this could not be a one-off, that we couldn't just get the answers we were looking for in one day or one day and a half. And for us to get the information and the knowledge that we would need to have a successful disease understanding experience if you will and which we knew would change over time with the evolution of a program that we needed to continue this but we also knew that because we were taking a different scientific approach that if we had to start with a different group of people each time we'd almost have to backtrack.

So we felt that it would be beneficial to start a group together that would make a commitment of two years and they would grow with us and therefore, their ability to advise us on things as the need arose would grow as well and so we developed a charter. We developed an application process where people could apply themselves or they could be nominated by someone who knew them in the community. We spoke with physicians. We spoke with the leaders of patient advocacy organizations. People started to come forward and they said, "this sounds interesting, I would like to apply" and we went through this application process and we interviewed people by telephone and what we really looked at to create a patient advisory board where from these applications and

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these interviews understand where people were in terms of how they looked at their own disease experience, how they kept up on research and other developments about their disease and about the field of rare diseases in general, where did they live. These are US-focused initially where did they live, what were their ages and so that we could really get a swath of the community if you will and to really be able to look at the diversity that we knew exists.

It seemed to work very well and after two years people said, "I don't want to leave. I'm really enjoying this." And we said, "great then you can reapply" because we always wanted to keep things fresh and we also wanted to give people the opportunity to say, "thank you I've enjoyed this and I want to spend my time doing other things" which is great. That was back in 2007 and even currently we still have on our Fabry Patient Advisory Board, we still have one person who has been with us ever since. So that I think is the difference between a focus group or patients focus group, the market research that might be done and we do all of those things as well and we bring some of the same kinds of questions that we might bring to a focus group we'll bring to an advisory board. But the beauty of it I think now 12 years into it is to be able to look back and to see what we've done and over 10 years we conducted about 30 or so patient advisory board meetings. These have always been Amicus organized and Amicus led events and that's where I think our hallmark lies is that we have incorporated the perspectives of these people into every aspect of our work across all the functions and as you said we're now seeing this model or something similar to it be adapted at many other companies and I think that's wonderful.

I will say the other thing that I can look back on is the number of people from each of the advisory boards we've had who passed away over the years. And it just drives home the urgency that there is in trying to help people living with these diseases. We have lost people living with Fabry, with Pompe, with epidermolysis bullosa. It's just the reality of these diseases and it's something that we always have to keep at the forefront and that as much advice as they're willing to give they may know that not necessarily where we're working on but they may know that there may not be something to help them yet but they're willing to share their experience if not to help themselves then to help the next person. And I think that that's something about the rare disease community that is inspiring and really beautiful.

Taren: Certainly a generosity of spirit. Your Good Stuff Program, talk to me a little bit about that one too.

Jayne: So that's definitely generosity of spirit, I'll tell you why. I don't even remember the year. They sometimes kind of wish together but I was at a family conference in the

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UK for families living with MPS, various types of mucopolysaccharidosis as well as Fabry disease. And then one of the breakout sessions at the conference, this mom whose daughter has Fabry disease start up and she talked about how to help your kids be positive when you're living with the challenges of a rare disease as well as the other challenges of modern family life – economics, relationships, school whatever it might be and she wanted her children to go to sleep at night thinking something positive with the notion that if you go and sleep with a positive thought or attitude you'll wake up with one. And so when the kids were little she would say, "what's one good thing, what's some good stuff that happened to you today?" And they would talk about it. As the kids got a little bit older she had a jar just a very simple Mason jar in the kitchen and she'd say, "here's some notepaper if something good happened, write it down put it in the jar."

So for her son who played across the UK so football, played soccer had scored a goal, write it down put it in the jar. A good exam result put it in the jar whatever it might be, a sunny day and then they would periodically empty the jar and read the good stuff. So that whatever the challenge or the disappointment that may have been there earlier in the day they could balance that out and as the kids got older they still put the notes in the jar but they would dump them out less frequently sometimes doing it on the afternoon of New Year's Eve where they can actually look back on many months and saying, "no matter what difficulties or bad stuff that happened. Good stuff is always happening too." So I sat there and listened to her presentation very basic, very simple and so direct and I just sat there with tears in my eyes and I said, "this is wonderful. More people should have the opportunity to think about incorporating this into their family's life." As the result reached out to the leader of the patient organization of course and said, "this is something I'd like to do. I would like to speak with this mom. Would you be able to arrange it?" And she did. This is the UK MPS Society and they arranged and put us in touch and I spoke with the mom. Her name is Margo and I said, "I would like to take your idea to other people. Do I have your permission?" And she said, "absolutely, " and we did what we needed to do and she and her daughter who was 15 at the time they were advisors to us on the project and we developed a brochure and we developed different kit with a notepad and stickers and things for families and it became something that we make available to people living with rare disease and it's really an exercise in positivity.

When we would talk to our patient advisory board about it when it was in development I remember one gentleman who has a son with late-onset Pompe but at this point was already out of the house and independent, a very successful talented young man and he said, "boy when my wife and I sit down and have a glass of wine at night we should talk about Our Good Stuff." And it really is something that anybody can use. You don't have to be living with a rare disease or any type of a disease but I think in today's world we all need to stay as positive as we can. And it's been very well received by the patient

community. We've had people who have asked us, we've had nurses at some of the centers we worked with they've asked for the stickers. They've them put them on their water bottles that says, Our Good Stuff and internally here at Amicus at our offices in New Jersey as well as our headquarters and our international headquarters over in the UK we have Our Good Stuff jars around and we encourage people, what's something good that happened then we take those periodically and we'll put them up on a monitor we'll digitize them and we've even had them taken the program to camps and to conferences that organizations are doing and they have a jar and people during the course of a couple of days of the conference put down write a note, put in the good stuff and then we've taken them and we've turned them into posters and we've given them back as gifts to the organizations. I think that we all need to remember our good stuff.

Taren: I think that's an excellent positive life hack that no matter who you are you could employ in your own day-to-day life and I'm going to find myself a Mason jar and I'm getting out the sticky as soon as we're done. Your Good Stuff Program is just one way in which you are impacting positively, the culture of Amicus. What are some of the other things that you're doing to bring that patient focus internally and really nurturing that kind of culture within the organization?

Jayne: The culture here at Amicus I think is quite special and it is because of our patient dedication and that it's been in the fabric of the company since we really started. It's something that we don't have to be told about. It's something that I think people feel and it's something that we make sure we revisit on a regular basis. So certainly the patient advisory boards I mentioned that's the program the Patient and Professional Advocacy has developed but it's a program for all of our colleagues across all of the functions. It's their agenda items. What did they want to hear from patients or caregivers that will help them in the deliverables they're working on, so people hear from our PAB that way. We've had lunched and learned since 2005 Lunch and Learns which a lot of companies have is the opportunity to bring in people who are living with the disease whether it's an individual, family, the leader of an organization, in our case, it's policymakers periodically and it's really to hear their narrative, what is their story, and what do they want to do to make a difference for themselves and for people that live with their disease.

So often there are many patient advocates, family advocates that want to have the opportunity to share their story because they're increasing awareness about the disease. They want people in industry to have a good understanding of their disease, what they live with, what's the unmet needs are because they're looking to our industry to be innovative and to make a difference. And what we do is we have people come in and they tell their story but also we've been doing lunch and learns that are thematic that we may have a panel and we may have representatives from different disease communities

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but we're looking at a particular issue. So the issue might be around caregiving and what are the needs of rare disease caregivers? And we did that last year and we tied into a project for the National Caregiver Association that we helped to underwrite along with other companies and it was a project done in collaboration with Global Genes to understand the rare disease caregiver experience.

We've had panels about for teens but we've had teens come and speak but they're talking about their experience living as a teenager with rare disease and what are the transition issues around school and around social aspects and what do we need to know about that. We've even had programs here at Amicus where we've explored our own experience with rare diseases and what I mean by that, we say that Amicus, we are a rare company but rare isn't rare here. It's common because we have many people who live with the rare disease themselves and have a family member. If one in 10 Americans lives with a rare disease and is affected by a rare disease and you've got a company of now we're well over 540 individuals you'd have people who themselves live with a rare disease and so we've asked people to share that. They made the diseases again that we're not working in, that we don't have a program for but there are certain commonalities, the humanity of living with rare disease which is so important for us to remember and to think about every day.

We also have a wonderful portraiture gallery here at Amicus and then all of our offices, it's the living in the light portraiture gallery where it's photography of individuals, of families living with rare diseases and in this case most of them are people living with rare diseases which we have a strong interest in or program in but it's also their narrative. It's not just who they are and when they were diagnosed but its how are they living in the light of themselves as a person. They're a person with a disease and they don't want to be defined by their diagnosis and I think that's something you hear more and more. We all are understanding the importance of storytelling whether it's in our industry or anywhere where people we need to relate to each other and I think that is one of the things that we've been able to do very well here. It's not a trend and as we grow and we have people coming from many different company experiences in different cultures one of the things that we look for, that we recruit for is the fit for this culture.

It's what we do and we bring the patient, the community to the table to be our partner and as patient advocacy leading this aspect of our work we actually have the obligation to be the conscience of the company and to make sure that we don't stray from our true purpose which is to make a difference by developing innovative medicines for people living with rare metabolic conditions.

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Taren: It's wonderful. It's very inspiring which you all are doing and I think it's really the work of heroes, so kudos to you and your team. We've touched on a number of challenges in the rare disease space there is access to medicine, there is funding, there is the research and development piece of it. If I were to challenge you to identify the number one area that you would like to see fixed, what would that be?

Jayne: That's a very interesting question because to say that something is a challenge doesn't always mean that it needs to be fixed. It might just need to be overcome because something that needs to be fixed implies that it's broken. I think that we're very fortunate that rare diseases are far more acknowledged than they used to be. They're of interest in the mainstream now. It's not as hard to get industry, to get academia and research interested in rare diseases. In fact the Rare Disease Day in February which is February 28th or 29th this year but the Empire State Building was lit with the rare disease college for Rare Disease Day, so that's pretty cool. So the recognition is there. I think the challenge continues to be that we are dealing with very small populations of people who are geographically spread out all over not just our country but all over the world and how do we connect with those people and I don't just mean we, Amicus and we, patient advocacy but how do we as drug developers connect with those people so they have an awareness of what might be available for them – clinical trials, access to faster diagnoses, knowing that people care for them and care about them I think that that continues even in this age of massive social media and technology that continues to be an issue because people with rare diseases are flung far around the globe and I think that it's that connection that continues to be a challenge for many of them.

I think the other challenge and I think it's a challenge that we're working at very well here in the US and in Europe is trying to be innovative in terms of the regulatory pathway, trying to take all of these differences between rare diseases and common conditions into account and look at being creative innovative in how we design clinical studies but also be truly cognizant of time. I mean time is the enemy here for these people. As I mentioned we work with people in our advisory boards but several of those people aren't with us anymore and as we work more and more in these diseases of neurodegeneration diseases of the central nervous system in infancy and early childhood time for sure is the enemy here and at the same time, we have to be kind to ourselves and we have to do things the right way. We have to do things thoughtfully and innovatively and we need to take the time to do it that way. So I'd say that that's a big challenge and something that's hard to always balance because you've got this sense of urgency and yet things have to be done in an appropriate regulatory environment.

Taren: That was a wonderful articulation of a couple of the areas that are at the forefront right now in the rare disease space. To switch tracks just a little bit, you are not just an

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innovator within the Amicus Therapeutics world but you also founded the Professional Patient Advocate and Life Sciences, I guess the acronym is PPALS and you also serve on a number of nonprofit boards and are associated with a number of nonprofit organizations. Why is this type of engagement important to you?

Jayne: I think Karen when we look at patient advocacy it is a growing area of our industry and we certainly recognize that years ago myself and my long time colleague and friend, Jean Campbell and Barbara Wuebbels, and we saw that people were coming to patient advocacy as I mentioned earlier from all different kinds of backgrounds. It could be a second or third career for someone who started out in marketing or sales or they started out in communications, with people who started out in regulatory and somehow they came to and they found patient advocacy and that's a wonderful thing and what we often saw was that there wasn't any one professional association or society where there could be some standardization of best practices, of how to do things, what kind of a background do you need, or how you tweak a certain kind of a background to become an effective patient advocate in the life sciences industry. We did a lot of due diligence about three years we did actually looking at all of the other functions in life sciences and the organizations that existed for them for ongoing education, for networking to help with job placement things of that nature and we didn't see it for patient advocacy.

That's why we started the nonprofit PPALS, Professional Patient Advocate and Life Sciences to provide that forum and to provide education and phase-to-phase courses of study not a conference that's run by a for-profit meeting planning company and although some of those are very good and they have their place, this is different, this is a non-profit with education at its core. We are now headed into our fourth year of course of study we'll be in May again at Stanford Research Institute out in Sioux Falls, South Dakota. We've had a very good partnership with Stanford where they work with us to not only make the course of studies available but to make it available in just an amazing conference center they provide infrastructure. It's been a wonderful partnership and in fact this year we are offering our additional level.

So if you think of 101 sort of an introductory level that's what we've had so far for people in industry as well as for leaders of non-profit organizations to help them learn around development and sustainability of a nonprofit but then how do you work with industry in the drug development realm.

This year for the first time we will have a 201 level if you will for both the non-profit and the industry participants for people who have come before they're now going to come

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and they'll be coursework on things that we weren't able to address in the introductory level. So we're very excited about that and I mean that is one way for myself to give back but also where advocacy has become so diverse and we feel that there is some standardization that could be helpful and that there are some people who've been doing this for a very long time that there's a new generation that's coming along and can learn from what has worked and can bring their innovation and their creativity to the field. So that's a big piece of it. It's to professionalize if you will, function that a lot of companies either don't have or had only recently we're very fortunate here in Amicus that it's been with us from the beginning, it's part of our DNA but also to advance it and preserve it so that people look at advocacy as something that's there for the patient.

It is not a commercial function for example, it is educational, it is supportive; so that's one thing and in terms of other non-profit work, I have thoroughly enjoyed my time when I was on the board of NORD, the National Organization for Rare Disorders before I got into industry, work I've done with Global Genes over the years. Certainly I've been very involved here in New Jersey with BioNJ with their patient advocacy committee and with a range of programs that they have been offering and that range is increasing and I've been very involved in different work streams through Bio particularly around government affairs and public policy and the HealthCare Institute of New Jersey and other organizations and what I find is that uniformly the organizations are recognizing the importance of patient advocacy and that they are providing forums and networking to help their member companies. So it seems that it's really this perfect storm right now between the non-profit side, the industry side, and organizations and associations that recognizes that advocacy is an intrinsic part of what we do.

Taren: Well that's fantastic and this would be a perfect moment to recognize the fact again that you were honored with the Second Annual Heart of Bio New Jersey Award for your dedication and selfless work on behalf of patients but that's just the latest of a long list of industry honors including their rare champion and advocacy nominees from Global Genes, you were named executive mother of the month by the bluesuitmom.com organization and that's just a few. That's a lot of star power but you bring a lot of star power to the game. As such I'd love for you to talk to me about what is one accomplishment or a wow moment that helped shape your career?

Jayne: Wow moment that's a tough one.

Taren: I know there's probably many.

Jayne: I think for me there are a couple that I think about and maybe they're a little bit more recent but I think the ability to be part of something from almost the very beginning

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I've been with Amicus now it's almost 13 years. Our CEO, John Crowley came in 2005 and I, in 2006 but to really see what advocacy can do but to be given the flexibility and the freedom to create something not in a rogue way at all but in a way that is innovative, that plays to strengths, that plays to creativity, and to really help to build something and make it happen and when we got our first approval for our Fabry product from Migalastat known as Galafold in Europe in 2016 that was certainly a wow moment because we had worked for so long and so hard to bring a product to the market that could help as many people as they're appropriate for this medicine and that certainly was a wow moment. I would say that where it gets even more so is when I now look at the number of people who are part of Team Amicus and we used to be 35 or so, we would sit in one room and we would do our lunch and learns that way and now we're in a very large area and we stream live to our offices and other locations and you literally looking out the sea of people and I sometimes find that overwhelming because while you're part of the growth and your head is down and you're working really hard that's one thing and you know it. But when you actually are standing there and you look out and you see the number of people that have joined this mission, that's a wow moment. And then to be doing the same and experience the same in our international headquarters outside of London that' another wow moment.

So I think it's the growth and I've have been privileged to be part of that growth and to continue to observe it and to help shape it, that's a sure is a wow moment. I think the other thing for me which is the most recent, if I look back on the work when I first started in rare diseases as I mentioned with National Tay-Sachs and Allied Diseases there's a whole family of neurodegenerative diseases with at the time limited research, no clinical trials to speak of, not a lot on the horizon but it was also the earliest days of gene therapy and one of the diseases we worked in at the time and is still very much a part of the NTSAD community is Canavan disease, although not a liposomal disease, it's a leukodystrophy. But in 1998 there was a gene therapy study for Canavan disease that had started. So I was very involved with that and the families and the families that were participating in the trial and the researchers and making sure that the community was kept up to speed on their gene therapy research.

But from then and just about until now almost gene therapy has been something that people have worked out, that they have learned about, and that they had hoped for and that they had seen as a potential treatment if not a cure and it was always five years away, five years away, five years away. Gene therapy is here now. We have approved gene therapies. We're seeing the difference that gene therapy makes for people who are on an approved product, for people who are participating in a clinical trial, and in the third quarter of 2018 Amicus was able to expand its pipeline by bringing in gene therapy assets including clinical trial that was underway then for CoN6, one of the forms with Batten

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disease and by the end of the year we were able to initiate a second gene therapy study for CoN3 or Juvenile Batten disease and we have many other diseases that are part of the pipeline that could be treated in this way with gene therapy. It's still early but it's very very encouraging and so for me, to have started in rare diseases at a time when there really wasn't much if anything and to be where we are now where there is so much and so much encouragement and promise is full circle and for me that's a wow moment.

Taren: Jayne, your Mason jar must be filled with good stuff notes because it's incredible. I can't thank you enough for sharing your stories and your journeys and your vision for the rare disease space. Thank you so much.

Jayne: Taren, thank you very much. I really appreciate the opportunity.

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