MORE THAN A VOICE: HOW PATIENT ORGANIZATIONS ARE DRIVING INDUSTRY R&D FROM THE START

Announcer 00:00:02

Thank you for joining us. Please welcome the panel to the stage.

Ebony Dashiell-Aje 00:00:24

Good afternoon, everyone. My name is Ebony Dashiell-Aje and I lead patient-centered research at BioMarin, and I'll be your moderator today. But before we begin, I want everyone to think of someone, someone you love, living with a serious illness. What would change if they and the patient groups that supported them helped meaningfully shape research for treatment from the very first conversation about unmet need? We all recognize that lasting progress in medical innovation cannot be achieved in silos. In fact, for thousands of those living with complex chronic conditions, including rare disease, the gap between a promising treatment and an accessible one is shaped just as much by the way that patient organizations and industry work together as by the science itself. So, today, our panel of patient-centered organization executives will show how those partnerships are shaping development priorities, clinical trial design, and access strategies from the start, and why patient organizations are more than a voice—they're a partner. All five of our panelists are a part of the 2026 to 2027 cohort of FasterCures LeadersLink Program. And this is Milken Institute's program for leaders of nonprofits that fund or conduct biomedical research. Through their capstone projects, every leader on this stage is actually building an industry partnership right now. So, let me introduce them. We have Manuela Battaglia, managing director of INNODIA, a global partnership in Type 1 diabetes that builds systems connecting research, patients, drug developers, and health-care systems. Then we have Jason Curry, a founder and executive director of the SCN2A Foundation, a patient-driven nonprofit accelerating therapeutic development for SCN2A-related disorders. Then we have Laura, Laura MacNeill. She's the CEO of CMT Research Foundation. In rare disease, the foundation gets resources, focus, and momentum behind the science that patients can't afford to wait for. Then we have Sudhir—sorry. Apologies, Sivakumaran. I said it. Yes! Had it phonetically, too. Is chief scientific officer of the Lewy Body Dementia Association, which works to accelerate therapeutic

development and improve outcomes for people living with Lewy Body dementia. And, then, last but not least, Emily Taylor. She's the CEO of the Solve ME/CFS Initiative. This unites scientific discovery, advocacy, and partnerships to make ME/CFS, Long COVID, and other infection-associated chronic conditions diagnosable and treatable. So, thank you all for joining us, and let's begin our discussion with Jason. So, Jason, we know every good partnership starts with a problem that not one side can solve alone. So, take us back to the beginning of your foundation. What did you need that you couldn't build on your own, and when did you realize that you needed a partner to be a part of that answer? What did you learn?

Jason Curry 00:04:11

So, when we started our foundation, it was because there wasn't enough support for the larger side of the disease. And how that partnership started, which—we've been supporting the gain-of-function side, so, which—gain of function is, for SCN2A, very severe epilepsy. And so, to explain what SCN2A is, it's the single largest genetic cause of autism, and it is the one, two, or three largest genetic cause of epilepsy, depending on how many papers they read at the time and which way the wind blows. I'm not sure. But we initially partnered with industry because they helped my son. We almost lost my son. They gave us the wrong medication. And when they helped my son get on the right medication, even though they weren't necessarily working on it, I just made it a mission to work with them ever since. And so, a lot of the work that we've been doing is helping them build infrastructure to find the right people. I have a background in emerging tech and growth marketing. And in three, four years, we've gotten two drugs to advanced-stage clinical trials. We have a small molecule that should be FDA-approved in December. If there's any FDA folks in here, give us some help. And then we have an ASO for severe epilepsy that is in our third-stage clinical trial that we're enrolling in Embrave Praxis, if anybody could give us some help? But that's a lot of how we've been working with our partnership and industry.

Ebony Dashiell-Aje 00:06:11

Well, it sounds like there's some really good success in the partnerships, but we know that that's not without friction as well. So, when you think about coming to these partnerships, and expectations and objectives of the different parties, where do you find that there might be still some friction between the different objectives and the outcomes that you're looking for?

Jason Curry 00:06:33

That's a good question. Us as rare disease founders, and especially myself, we give up everything to do anything we can to help our kids. It feels like we owe it to industry to just do whatever we can whenever we want, and it's just not feasible. And the amount of value that we provide and produce—we've gotten a whole handful of folks enrolled in ASO, which is an anti-oligonucleotide therapy, which is very precise for a very small amount of people, and we've enrolled several people globally and had them flown in at a fraction of the cost of what they're paying everybody else, but do we see any of that value coming back to us or that investment? No. So, the amount of value that we provide is substantial. And is that reciprocated? I think it could be much better, reciprocated much more—at least pay us market rate.

Ebony Dashiell-Aje 00:07:45

Well, thank you, Jason. That tension is actually at the heart of this panel. Patient organizations, they bring trust, urgency, and insight, but yet too often may feel that they walk away with the least from the partnership. So, now let's turn it over to Emily. Emily, you lead Solve M.E., and you're a caregiver, so you

see both sides. Where's the biggest gap between what patients need and what research and policy might deliver, and how have you learned to connect the scientific discovery, advocacy, and partnerships with what you're trying to solve at your organization?

Emily Taylor 00:08:20

Oh, big question. Yes, I am a caregiver—oh, first of all, hi, everybody. My mother has ME/CFS and has had it since 2008. My brother has Long COVID, has had it since 2022. And so, when I approach these circumstances, it is as a caregiver, and it is with a very personal touch with these relationships with pharmaceutical companies and government partners and other major industries. When I think about the key elements that make a successful partnership, especially when you're bringing the patient community as a whole along with you, and it's not just my mother and my brother, I think the key elements are, well, everyone talks about funding. That's sort of the elephant in the room. Funding is critical for small organizations like ours, and especially when big pharmaceutical companies utilize our resources, not at market rate, because we are so willing to give them. We're getting that money from somewhere, or it takes us away from some other work. So, that's the first one. The second one is transparency. Transparency, transparency, transparency. I cannot emphasize this enough. So much that happens behind closed doors, in private conversations, in boardrooms, and business conferences doesn't get back to the patient community at large, especially the patient community that doesn't have the capacity or bandwidth to be reading every article and following every newsletter. So, having that clear transparency with the community about what's going on, who's in the room, why decisions were made the way they were, is absolutely critical. And last, as was already referred to, true, equal partnership: valuing what each organization brings, whether it's a pharmaceutical company bringing billions, whether it's a patient advocacy group bringing the voice of thousands, whether it's an individual who has the immense resources of experience and lived experience that they can bring to that conversation. So, I think those are the three elements that make for good partnership.

Ebony Dashiell-Aje 00:10:10

Wonderful. Now, we're going to move to Manuela. Manuela has a different perspective on this panel. INNODIA acts as a bridge organization, right, Manuela, between patients, researchers, and industry? What does that bridging make possible that neither side can build alone?

Manuela Battaglia 00:10:31

Yeah. So, INNODIA is a European multi-stakeholder platform that brings together, as you said, the different stakeholders: clinicians, scientists, and patients. And we make sure that they understand each other, first of all, and that they speak the same language, or at least they learn how to speak the same language, because every component has different incentives. They have the same goal: they want a cure; they want therapies, but they are incentivized in different ways. So, the platform that we have built has the goal to make sure that the different components talk one to the other. They have opportunities to meet. We organize a lot of events online or in person where we make all these components, talking one to the other. We have built a training platform for patients where they can learn a bit about what it takes to bring a drug from research to clinical development to market access, all those steps that often patients are not aware. And so, with that, we empower the community and make sure that their voice is not just made of lived experience, but also of some knowledge. Because what we think is that a voice to be heard needs, of course, the experience, but also the right language and the right understanding to sit at the table. You

need to understand the table you are sitting at. And you cannot expect that everybody listen to you if you don't speak the right language and if you don't know the process and the path. So, I think that an organization like INNODIA really helps in reducing, first of all, fragmentation among patient, patient community. In Europe, I don't know how many patient organization working with type 1 diabetes patients are there. There are thousands. So, we try to bring them all together and make one big voice. And also, we represent the different social impact that this disease has. If you live in Italy, you have access to certain therapies, certain technologies. If you live in Lithuania, you have others. So, these are different voices that needs to be represented. And also, as said, we add a bit of a knowledge and have expert patients to the table. So, I think alone, this could not happen.

Ebony Dashiell-Aje 00:13:20

Wonderful. So, you identify what the real need is, and now you've created this framework and also this hub so that folks can come together and be a community together and bridge the needs. Now, let's move on to partnership design. So, Laura, I'm going to talk to you. Jason, he named that imbalance in some of the partnerships that you might see between industry and patient groups. And Manuela showed us what a bridge makes possible. But we know that what bridges is contracts and different infrastructure. So, how are building your partnership strategy and vetting partners? How do you guys do that, and what goes in writing so that patients and the organizational reputation and interests are protected?

Laura MacNeill 00:14:16

Yeah. I get the fun part. Hello, everyone. At CMT Research Foundation, we're only an eight-and-a-half-year-old foundation. CMT is Charcot-Marie-Tooth disease, which is a disease of the peripheral nerve, first described 140 years ago, but there's no FDA-approved treatment on the market. So, our foundation was exclusively designed to really have a high-impact hit on getting treatments to market. So, we weren't set up to do the traditional model of patient advocacy groups, which might include things like patient services and education and today support. We really have a narrow focus. So, out of the gate, we were designed under a venture philanthropy model. So, every grant that we issue has an ROI return, and that requires a little bit of a different process and a very different management style when we're working with industry, which we consider our partners. So, our venture philanthropy grants range from academic partners all the way up to—we've manufactured a gene therapy now. Every deal we approach very carefully. This includes your traditional sort of LOI process and then a vetting process through a scientific advisory board. All of those are scored and gated. Then it goes to a research advisory council. That group's really a group of drug development experts from industry who are looking at that project and trying to qualify the path to clinic. We're trying to de-risk it while we're evaluating it. So, our traditional grant, over the course of when we first get one, an application for revenue, to when we fund it, our average savings and cost is 17 percent. So, we're really working as a partner there, saying, "Hey, we have access to maybe a lab service that can bring this down," or "We have experts that might be able to do this for a little bit less cost." And so, when we get finally to an agreed-upon partnership, which is approved by our board of directors at the end, it then goes into a contracting stage, which can take anywhere from six weeks to six months, just depending on what we're working with. So, all of those look a little bit different. Of course, your standard package is going to have an NDA. Everyone feels more comfortable with an NDA. And then we go through a vetting process to try to design what that ROI is going to look like, and for us, that can really have a wide range depending on the partner.

Laura MacNeill 00:16:40

So, this could be something like royalties, which we all know about from the Cystic Fibrosis Foundation, but we might have convertible notes, and we negotiate that. All of our projects are milestone-gated, and so, we work with our scientific experts to say, "All right. We'll do an upfront investment in this total grant, and then by this stage, scientifically, where should we be to understand that the follow-on funding for the rest of that grant is de-risked? The project is on track?" So, those all get established with the dollar amount and the scientific milestones. They're preset out. It's a contract done with a lawyer, a legal team, so we're working in-house and out-of-house to get that designed. And then, as the project goes, we execute those milestones, evaluate the project, see if they're on track. If they are, we continue funding. If they're not, we stop funding. It's built into our contract to be able to say, "Best intentions. We all thought this would work. Early work, this is showing it's not on track. We're going to return that money back into the system and put it towards another future project." Throughout all of this, Ebony, we're communicating this out to our community. We are often, as patient advocacy groups, especially when we're funding medical research, translators. We are right between industry and the patients that we serve. And so, we are frequently doing lay-friendly blogs. We have a monthly webinar series: we'll bring in the key investigator we funded and ask them to speak directly to the patient community with our guided Q&A to make sure everyone understands the project, the potential outcome, the impact on the community. And we continue to do updates, including when projects don't go the way we anticipated. We have a really strong feeling that that should be communicated as well with the successes.

Ebony Dashiell-Aje 00:18:28

If we can hit on that note, when things don't go the way that we expected, the whole returning back funds, et cetera, how do you bake in the transparency upfront so that all the parties understand what the outcome could be, and how do you sort of mitigate the friction that could happen in that process?

Laura MacNeill 00:18:50

Yeah. So far, we haven't had friction. Last year, we returned a little over half a million in promised grant money that just—the project wasn't on track. I think you do that when you're negotiating the upfront milestones. So, that is both sides saying, "This is scientifically how this should progress. Here's where we'll put a gate and say, 'Okay, we should be able to measure progress at this point,' and then this is the amount of money that will unfold after that." And so, the report that they return in, which is a milestone report, is pretty thorough and conclusive, and it often doesn't turn into a "But you owe me." It's best intentions and let's try again. Let's start over. No one wants to waste time and energy and resources on a project that just isn't going to get a drug to a patient. And we're very upfront that that is our entire purpose: to get a safe and effective drug to market for Charcot-Marie-Tooth disease.

Ebony Dashiell-Aje 00:19:41

Last question for you.

Laura MacNeill 00:19:42

Okay.

Ebony Dashiell-Aje 00:19:44

So, for an organization who is looking for that funding, what would be one key piece of advice that you have for them to plan for being able to hit those milestones and have success? What partnerships do you feel that they need to have to ensure success from the very beginning?

Laura MacNeill 00:20:00

Yeah. It's a great question. Most projects that come when they put forward their full request for funding really lay that out thoughtfully. And when our scientific advisory board and research advisory councils look at it, that feedback loop is constant. So, we're helping to negotiate to say, "This is how we think you're going to unfold," and they're partners there. I think there are times where we do create what we call a JRAC, which may be a familiar term to the room, but a joint research advisory council. And this is where we feel like maybe the group has a great idea, but they don't know Charcot-Marie-Tooth disease as well as they should. We help to de-risk it by putting the right experts from our side on a joint committee with their team, and that's going to be a project that that team might meet monthly versus waiting for a milestone, which could be months or even a year and a half along. We're meeting more regularly to make sure that we are staying right on top of a project that might be high risk, high reward, extra complicated, or again, they might have a delivery technology, but they don't have expertise in our disease, and so we help to de-risk it that way.

Ebony Dashiell-Aje 00:21:06

Wonderful. Jason, from your experience, what's just one thing that you would advise a patient group to have as their tool to be able to level the playing field when it comes to trying to seek funding? And think about templated things, SOWs, the NDAs, the contractual pieces.

Jason Curry 00:21:28

Yeah. So, one of the first things that I learned very quickly was—when we initially got our stem cell line created, we signed it and didn't have any of the rights. And I'll say thank you, Chris Moxham from Transcripta Bio, for signing that all over to me. We were very fortunate. I spent a lot of time just calling lots of large legal firms, and—shout-out to Morrison Foerster, that they signed us up and have just been absolutely amazing. And that was really our big unlock. They provided us with—NDAs are fairly standard; they're a little tricky in this space, for us at least—so, NDAs, material transfer agreements, your MSAs, SOW template. I have a business background, so I understand some of that. It's a little bit different in this space, but I've just seen how much that completely wipes out a lot of new foundations, or it sets them back years whenever—if you lose all of your leverage, you don't have anything to stand on, and then nothing moves forward. So, if you can provide those assets upfront to a lot of these beginning foundations, that they're putting their life into curing their kids, you just won't find anybody that has more drive to solve that disease than that foundation. And if you support them, they will take it further than any other investment.

Emily Taylor 00:23:16

I would be remiss if I didn't mention there are three tools that are fantastic, and I really want to give a shout-out to the groups that have made them. The first is the Patient-Led Research Collaborative's patient engagement scorecard, an excellent resource. Highly recommend. If you're looking to engage with a patient community, that scorecard is everything you need in how to utilize experts and patient advocates and lived experience experts together. The second is the National Health Council's Fair-Market Value

patient calculator tool. Highly recommend, again. You can literally just type in the services you're looking for, how many hours, and it'll give you a fair market value calculation. And the last is trained patient leaders. Our organization has a program called the Long Covid Patient Leadership Advancement Network, or LC-PLAN, and we find and elevate leaders who are already engaged in the patient community and give them the tools they need to serve on these advisory boards, to serve in protocol development, to serve in R&D, walk them through what is a protocol, what is the life cycle of a drug to market, what are these basic business skills or science skills that the average patient wouldn't have, but they have the leadership capabilities innate already there. So, those three programs are exceptional ways to get these tools to help these partnerships flourish.

Jason Curry 00:24:35

Can I jump in there? So, that's exactly why I launched RareDisease.com today.

[Applause]

Just go on your phone and check it out real quick. So, previously, I ran a growth consultancy for years, and we were the agency of record for a lot of large agencies. So, I took all of that data, everything that we've built, all of the infrastructure, all of the repurposed drug data, and built and launched two things today—scn2a.ai, you can check that out, too—but RareDisease.com. Why that's important is when you build a website, just something as simple as a website, there's foundational pieces of information that I think that we should own. What is this disease? What are the treatments? What are the symptoms? So, this platform that we built will spin up an amazing website for a foundation overnight and give them the leverage to sort of own that space that they can develop on. Along with all of those documents that we talked about. At least my versions of them. There's probably better ones. If you guys want to share what you have, it'll help everybody else out.

Ebony Dashiell-Aje 00:26:00

Wonderful. So, it sounds like partnership with the actual developers and industry aren't the only partnerships that are critical for building success. You have to have key experts in different areas, particularly to fill the gaps that your organization may not be able to fill, and make sure that you get those partnerships set in place upfront so that you're set up for success from the very beginning, and you're not sort of giving away some of the safeguards that would protect the progress in the future. Okay. We've talked about what patient organizations need going in. Now, let's flip the lens a little bit, talk about the other side of the table. What are companies actually looking for in a partner? Manuela, you sit in the middle of that table, so let's start with you. What demand signals are you seeing from companies, and how do you get patient participation built in when a company sees it as optional?

Manuela Battaglia 00:26:56

Yeah. The first thing they look at is de-risking their path and speed, speed, speed. Just help me in reaching my goal faster. And so, they usually reach us out when they start the clinical trial, and they think they need patients to put into a trial. So, that, I think, is the first goal for medicine developers. But then our goal is really the moment we talk to them to make sure they understand that patients can contribute to so many other different steps. Starting from understanding whether the preclinical package that they put together makes sense to move forward to a clinical developmental stage or, when they design a clinical trial, is that those inclusion criteria or endpoints or the operations around the trial—do they make sense? Are patients

going to accept it? When they write the informed consent, is that clear enough for the patient? Does the language is sufficiently self-explanatory, patient-facing materials? So, there are so many steps that where the patients can contribute to. And really, our goal is to make sure that they see the patient voice as a contribution so important and so key that they cannot live without it, and not just when they need to go fast. So, that, I think, is really changing the mindset of the medicine developers and see more as a holistic approach, I would say.

Ebony Dashiell-Aje 00:28:44

Wonderful. Punting it to you, Sudhir. Your work is about moving things upstream. So, where can patient and caregiver priorities change endpoint selection or trial design, and what has to be agreed upfront for that to be a success?

Sudhir Sivakumaran 00:29:07

Yeah. So, I think that's the big challenge. So, I'll start with a small anecdote without naming the companies. But after I joined the Lewy Body Dementia Association, for people who are in the space know that there is still biomarkers that needs to be developed, outcome assessment scales, and definitely therapies. There's no drugs for dementia or Lewy bodies right now. Even symptomatic treatment, they're still being worked on. So, we work with a lot of industry partners, and I asked a couple of them earlier this year in one of our annual meetings, on a panel session, actually. It's like, "Okay, what would it take for you to launch a trial, a development program? You have the money, you have the resources." We are talking about the global pharmaceuticals. They said, "Well, we need biomarkers, endpoints, and longitudinal data and outcome assessments." So, that's pretty much everything that we are looking for. So, I wanted to share that—that's an actual fact. I wanted to share that, not to criticize the company, but to actually identify—I see these as opportunities, and the title of the session, how do patient organizations or nonprofits are driving industry R&D? So, nonprofits, we don't do research and development. We don't have a lab, but we play a significant role. Partnerships with patient organizations should be strategic partnerships, and that's why they approach us. So, for instance, in drug development, I'm sure the industry colleagues will agree that industry doesn't compete for the tools; they're competing for the molecules. So, who develops the tools? It's usually those nonprofits, the patient organizations. And why us? Why not an academic center? Why not the Harvards and MITs of the world? It's simply because when you develop a tool for drug development, you need to bring multiple stakeholders together to the table. And you need to achieve consensus on the tools, on what the tool is or what the purpose of the tool is. And nonprofits and patient organizations are one of the best places to do it, and industry knows that, and that's why they still continue to partner with us, even though we wish the partnership could be much more than what it is today. But I'm sure it's better today than it was 10, 15 years ago. The one thing I want to specifically highlight is that, of course, the most common needs that we get at LBDA from our industry partners is communication of ongoing trials to drive recruitment, site selection, and increasing the number of patients and retention and all the aspects. But from a research side, we have our industry partners right now specifically asking us about what are the different contexts or the use cases for the various biomarkers that are being developed currently in DLB and dementia with Lewy bodies. They're still in development, and once it's developed and validated, you need to define a context in which a biomarker can be used. The same thing with outcome assessment scales.

Sudhir Sivakumaran 00:31:53

Right now, there is no outcome assessment scale for Lewy body dementia, and that's a fact. There is one that's currently being developed by a bunch of academics in Europe and the US, but still a lot of work that needs to be done. So, the industry comes in and says, "Okay, can you," you being LBDA, "help generate preliminary data, patient-level data, for these outcome assessment scales?" which will then give the confidence to the industry to think about a development program, think about a trial design, selecting the right endpoints. And finally, we bring the perspective of not just the stakeholders who we work with, but the main stakeholder, which is our patients and caregivers and families. I'll make one last actual fact, and then I'll stop, which is that—so, we had an annual meeting this year at the Lewy Body Dementia Association in Atlanta, and we had a session for individuals with DLB and their families and care partners who shared their experience with the disease and how it took eight years to even get diagnosed because of the challenges. And there was one colleague, a woman, who was diagnosed after eight years, and she mentioned one of the biggest challenges she found was that for eight years, they kept asking her to draw a stupid clock. So, for those who don't know, in dementia, if you go to a memory clinic, there's a clock-drawing test. You have to draw a clock. It's basically a normal clock. She said, "I keep getting asked to draw a clock." And she said, "If one more physician asks me one more time to draw a stupid clock, I'm going to lose my shit." Sorry for the language. That's what she said. Now, that matters. The industry needs to hear it. The FDA needs to hear it. Again, what matters most to the patients? What's meaningful? Defining clinical meaningfulness is a very difficult concept, and I cannot speak for someone else. We need to hear from that person. So, those kind of perspectives is what industry looks for, and we can provide them. And how it evolves into an actionable development pathway is for them to decide. So, those are some examples in which we are driving industry research and development from the early stages.

Ebony Dashiell-Aje 00:33:50

So, industry does seek elevating the patient voice to be able to drive endpoint strategy and measurement. But oftentimes, there's a view that patient partners are brought in too late, so the protocol is already drafted, or there is an opportunity to gather insights about unmet need, and they may do it at some point, but it may not be early enough. And we also see from the examples that are brought here, industry often calls patient organizations once enrollment problems happen. So, you mainly concentrate that type of interaction when you're trying to mitigate a problem. So, when we think about that and how there may seem to be a delayed involvement of the patient organizations, there may be a breakdown of trust then, if industry is constantly coming to patient organizations, asking for them to sort of rescue. So, when we think about that, we know that there's value of bringing patients as partners upstream. And so, I want to get into that piece about what's important to move the patient voice further up and true partnership, not just, "Oh, I want you to help me with recruitment." How can you truly influence the research design and process? So, I want to go to Emily. Can you give an example of just how patient input changed a program and what should have been agreed to at the start that could have helped move things more efficiently?

Emily Taylor 00:35:29

Absolutely. I'll give an example on both sides. I'll give an example of kind of negative behavior and then an example of positive behavior so you can kind of see the dynamics and the difference. On the negative side, we were called in one of those circumstances where an organization had run a trial, and their results were coming back really distorted, and they weren't really clear on why their results were so funky, especially with patients with ME/CFS and Long COVID at baseline. And so, we asked them to walk us through, "What were you asking the patients to do? What's your protocol look like? What was the experience of these patients?" And through that discourse, we discovered that the parking garage in which patients were

being asked to park in and then walk to the clinic was over 100 yards away. And you're talking about patients with energy-limiting disabilities, with post-exertional malaise, and a walk of that kind for these patients is devastating. It triggers a flare. It can trigger a crash. It can be really, really harmful. So, when we pointed out, "Well, of course, your baseline measurements are all out of whack because we are inducing symptoms before the patient even gets into the clinic to get their baseline examined." So, we suggested bringing it home, giving the patients wearable devices, and letting them get their baseline at home, and then doing things at the clinic, offering wheelchair assistance or a golf cart that can get them from point A to point B without exerting themselves and pushing that energy envelope. So, that was an example we brought in too late, but had we been brought in earlier in the process, that would've been identified and patients would've avoided all of that harm. Now, on the positive side, a really great example is our partnership with the organization BioVie, which ran the ADDRESS-LC trial. Before the trial was even an infant, before it was even a sparkle in someone's eye, we were already in conversations with this organization about how to work with this patient population, what those unique needs are for folks with energy-limiting disabilities, and we also advised them on what funding opportunities there were. And we were able to help advocate for their inclusion in the Congressionally Directed Medical Research Programs, to get Long COVID included as an eligible topic area of that program, before they even submitted the grant, which then they received $13 million to run the trial. And we were part of that, not only in protocol design, but also in recruitment support and dissemination, where they're at right now because their results are out. Highly recommend you Google it. Really exciting work on the drug, I believe, bezisterim. I'll pronounce it right one day. But, yeah. So, there's two examples of how this can be a true partnership with benefits at all levels of the process for both parties, or you can come in that moment of crisis and your data is already gunk. So, highly recommend the former rather than the latter.

Ebony Dashiell-Aje 00:38:13

Wonderful. Punting it to you, Sudhir. Your work is about moving things upstream. So, where can patient and caregiver priorities change endpoint selection or trial design, and what has to be agreed upfront for that to be a success?

Sudhir Sivakumaran 00:38:27

Yeah, I think it should start from almost a challenge to our industry partners, with giving them the benefit of the doubt by acknowledging the concept that—we keep hearing about the voice of the patients and voice of caregivers and incorporating them, but I think incorporating the voice of the patients and caregivers should be a design principle and not an afterthought. That should be a design principle.

Emily Taylor 00:38:50

Clap, clap, clap for that.

[Applause]

Jason Curry 00:38:53

I was waiting to jump in and say that. I was waiting, waiting. How is that always missed? Geez.

Sudhir Sivakumaran 00:38:59

I think, and I strongly believe in that. Neuroscientist by training, but I have been in the nonprofit space for almost 10 years now, and that's something I've learned. I was not born with all this wisdom or genius, I think. But that's something I learned. And so, I think, first of all, that needs to be acknowledged by the industry, not just as a check mark of saying that, "Oh, yes, we engaged the patient organization," but it's a meaningful partnership. And moving upstream, once again, if you look at Alzheimer's and Parkinson's disease, tremendous progress has been made. But if you go back and look at some of those scales that they use for those monoclonal antibodies, some of the drugs that are developed, or all those development programs, they included significant amount of patient voice and as much as possible, as early as possible in the development program driven by those big organizations. But yes, now we have the small patient organization. So, there is a little bit of that perception, I'm sure there is even though they might not say, it's like, "Okay, what can a small organization like LBDA do to my multimillion-dollar development program?" The thing is, see, we are small, but we are mighty, right? So, we have PhDs, we have scientists, we have advocates. We know what it is. So, we can help it, to some of the elements of recruitment and retention, endpoint selection, biomarkers, assays. There are various ways in which we can play a role by moving upstream into the development process. So, when industry is thinking about scientific and clinical evidence that they need to generate, or even the regulatory evidence that they need to generate, that's a place where patient organizations, I'm sure, can play a role. I'll give a quick example and stop then. So, going back to the outcome assessment scale that I talked about—I just remembered that I didn't say what the scale is. It's called LBD-DRS, Lewy Body Dementia Domain Rating Scale. A paper was just published, too, a couple of weeks ago. So, this scale, again, it reads lots of qualitative data. So, moving the engagement with LBDA upstream to the industry sponsors who are developing drugs for Lewy body dementia, or dementia with Lewy bodies, is going to be tremendously helpful because we can bring qualitative data on what the scale is showing right now through our centers of excellence, which the industry can then take and then use it to design your trial, design your developmental pathway, and design—when you think about measuring something in patients or measuring something to quantify the treatment efficacy of a drug, you need to know what to measure, when to measure, and what it means when you're measuring something. And those are the information that we can bring. And again, this cannot happen after a phase one trial is done and the data has been collected. It has to be done before the trial is even designed or during that pathway. So, there are various ways in which patient organizations can play a significant role in drug development and clinical trials.

Ebony Dashiell-Aje 00:41:56

Before I pile on to that, I want to remind the audience, brief break, please scan the QR code if you have any questions, because when we go to Q&A, we want to be able to capture your questions on the iPad to answer. Back to the regular program.

Jason Curry 00:42:16

Can I jump in on that real quick?

Ebony Dashiell-Aje 00:42:17

Yes, you can.

Jason Curry 00:42:19

So, if it's called clinical trial design, how many actual designers, like user experience designers or system designers, are actually working in that process? Because I haven't seen any. I have a background in design. I haven't seen any. You have molecular biologists, you have medicinal chemists that are designing a process and designing it to be as absolutely complicated as humanly possible. So, if I could just say anything: hire for specialty and deep expertise. Go to Parsons, go to some design schools, get really good user experience folks or people that understand systems design, and bring them in. Pull the scientists out of that process. And designers just naturally make things more efficient. So, that's all I'll say about that.

Emily Taylor 00:43:17

I'll just piggyback on that and add that patient advocacy groups and patient organizations are those critical partners. If you get an advisory board of 12 patients, you have 12 experiences, 12 voices, a good start. But a patient organization, like Solve M.E., like any of the folks represented here, brings thousands of voices. They collate all of the data of the community and bring not just one voice, but many. And that's why patient advocacy organizations and patient organizations in general are critical and should be in that design role as well.

Ebony Dashiell-Aje 00:43:48

And if I could just echo from former regulator, current industry partner: it is very important for us to think about how we can incorporate that patient voice into endpoint design upfront. Because I think there is a high rate of failure when you do not incorporate what things actually are supposed to mean, or whether or not those concepts of interest tie back to the actual lived experience of those who have the conditions. And I think when companies try to speed, and speed to market, speed through the trial, we know that that also can benefit patients, because the quicker you can get a drug on the market or a device on the market, the quicker someone can have access to it. However, we can't cut corners with the patient voice. We do have to make sure that that's embedded in the DNA of what we do in order to achieve success. And we know that policy supports that, too. Our regulators have embedded these expectations into their benefit-risk review and their benefit-risk assessment. And it's critical for success of just a drug approval to be able to have this evidence that's presented. And even for payers. We often talk about approval, but what about access? Payers are looking for patient-relevant information and verification that it's patient-relevant. But if you don't have the evidence to show them that it's sufficient, then you're also creating a barrier. Okay. Well, let's move on to talking about values, value alignment. And we heard a lot about what works, but we know there's some things around value that really are critical for alignment with the partnerships. And Jason, when you think about how you're looking for partners for your work, driving the innovation that you're trying to drive, how do you keep values aligned and also at the center of the different partnerships that you're trying to seek? Where do you hit a wall, potentially? Because there can be tons of partners, tons of people that show interest, and we talked about potential perception of one-sidedness.

Jason Curry 00:46:10

Sure.

Ebony Dashiell-Aje 00:46:11

Some organizations may have one objective, but it may not align with your objectives. How do you handle that friction?

Jason Curry 00:46:19

I don't know. I'm still trying to figure it out. At least for us, as a smaller organization, we are trying to create as much leverage for ourselves as we can. And when you first start into this process, it's, "Do everything for free. Help us in all ways possible," when we're dying to just get out the door in the morning. So, it would be beneficial for the industry, at least for our disease. It's a larger rare disease. We've built the infrastructure, the partnerships, the legal teams, and the technology to move our disease forward. We just need the money. My son was diagnosed when he was two, and he's nine. And it's all there. We just need somebody to invest in us to help us move it forward. So, I think that's the biggest thing. And however they want to do it, we're open to that conversation. But it can't be lie down on the floor and walk all over you all day long. We have to work together, and whatever those stipulations are, if you let us know, we will take care of it so fast, like nobody's business.

Ebony Dashiell-Aje 00:47:59

Well, thank you so much. So, we're coming to the end of our planned discussion. So, let's just do a quick lightning round. One sentence. What's at stake for your community if these partnerships don't happen, and what's the one thing that industry should do differently to help you? Let's start with Emily.

Emily Taylor 00:48:19

Oh. Nothing about us without us, period.

Ebony Dashiell-Aje 00:48:23

Love it.

[Applause]

Sudhir?

Sudhir Sivakumaran 00:48:29

Yeah, what's at stake here is, from the perspective of patients and caregivers, it's time, and time is something we cannot give back to these individuals. So, a challenge to the industry is: engage with patient organizations early when you think about generating the evidence that you need to generate for a development pathway, and we can help you in so many ways that you might not think about, but there are always opportunities. And once again, incorporating patient voice should be a design principle when you think about a development program, not later in the time. And acknowledge that this precompetitive collaboration leads to competitive benefits always, and that's a fact. Yeah.

Ebony Dashiell-Aje 00:49:06

Wonderful. Laura?

Laura MacNeill 00:49:08

Yeah. I think for CMT, it's a genetic disease, so we have generations that have already lived and had no treatment. We've had generations that are aging out now. When this is in your family, it's prevalent down

the line. And so, what we risk if we don't pull this together is we'll have just more generations that carry this burden when we know this is a solvable problem. The only difference between a treatment for CMT and where we are right now is just resources and focus. We understand the genetics. We understand the solutions. The science is ready and available. And so, for me, it would be another 140 years without any treatment for a disease that doesn't need to create the suffering that it does.

Ebony Dashiell-Aje 00:49:52

Jason?

Jason Curry 00:49:54

Can you repeat the question again?

Ebony Dashiell-Aje 00:49:56

Now I got to open my iPad again.

Jason Curry 00:49:57

Because I want to make sure I hit it really hard. What's at stake?

Ebony Dashiell-Aje 00:50:01

So, what's at stake for your community if these partnerships don't happen, and what's the one thing industry should do differently?

Jason Curry 00:50:09

So, what's at stake is my son's life. That's what's at stake. I was trying not to cry, but here it is.

Laura MacNeill 00:50:16

You didn't swear, though.

Jason Curry 00:50:18

Yeah, I didn't swear. Yeah. So, that's what's at stake is my son's life and all the kids that we're working for. Because they're dying, and we have everything put together, and we just need the money.

Ebony Dashiell-Aje 00:50:32

Thank you for sharing that.

Manuela Battaglia 00:50:35

So, yeah. For us, for medicine developers, is to make sure that they understand the value of patient voice since the beginning. And sometimes you need to negotiate a bit with them to give up something to make sure they learn that patient voice needs to be there since the beginning. And from a patient perspective, I

would say, “Let’s professionalize a bit their voice.” Meaning, on top of lived experience, let’s build some knowledge, become expert patients, so you become stronger and more recognized as a key stakeholder in the process.

Ebony Dashiell-Aje 00:51:23

Thank you all so much. That just gives a lot to build on. Let’s go to the audience questions now. Let’s see. This will be open to whomever wants to answer. How can we get industry to compensate patients for their input? I know, Emily, you want to answer that.

Emily Taylor 00:51:48

Yeah. Hold their feet to the fire. Demand it. They come to us in those moments of need that you described where things aren’t going well, and that’s when to start using our leverage. Ideally, we start an equal partnership at the beginning of the process, and I think enough of those examples of things coming at the end of the process in poor taste will start building that in. But we have power as a patient community. We’re their consumer. If we’re not going to buy this product, then why are they making it? So, we need the patient community buy-in from the lived experience buy-in from the beginning, and to stop treating them like an afterthought or a subject and start treating our community as their community, as our collective community, and bring down those barriers between these lines of thought.

Manuela Battaglia 00:52:39

Yeah. For us, this is the value of having a bridge organization, an in-between layer. For instance, in INNODIA, we pretend that patients, when they contribute with their experience, they’re paid, exactly as the big scientists, the big professors, are paid by companies. So, we reward the hours that everybody puts into their activity the same way. So, that’s what I meant with professionalize the patient voice. The moment you have expert patient, you should expect that they are rewarded as well as expert technical. So, I think this is so important, and we should not give up on that.

Ebony Dashiell-Aje 00:53:25

Wonderful.

Sudhir Sivakumaran 00:53:26

And may I just make a quick point to that? We talk about the financial compensation from the industry, and—this whole session, I do want to point out that it’s not like industry is not doing anything, and we are asking the industry to do anything more. Honestly, the industry is a tremendous place. I’ve worked with a lot of them, some of the best people I’ve ever met, and hopefully, at some point, I wish I could grow up like some of them. The industry is a great place. This is not a criticism. In addition to the financial compensation, one of the other things I’ve noticed is incentives to patients to participate in a study or continue participating can be more than just money. This was an actual data that was shown a few years ago in a roundtable in Washington, DC, where they did a survey of what would it take for patients to sign up to a trial or stay in a trial, and the first response was return of results. So, that’s another thing the sponsors can keep in mind, and everyone. Sometimes even getting those results back from the trial is going to be tremendously informative because one of the biggest challenges for anyone with these devastating diseases is the uncertainty and the unknown. Not knowing what’s happening. No one is telling

them what—no one is explaining to them what a result means. Even that goes a long way. And this was an actual massive survey someone did, and the first response was, "If I can get my results back, if I know what I'm going through, I'm more than happy to participate in the next trial that you're going to do." So, I do want to mention that industry, in my opinion, already does very good compensation. They are very good in that, and they do more than that to provide that value proposition and incentives to the patient and families and caregivers.

Ebony Dashiell-Aje 00:55:00

Thank you. All right, another question. Specifically in the rare disease space, where commercial viability may be limited, how can patient organizations position themselves as attractive partners to industry? How do they show their value in dollar amounts that they will respond to? ROI.

Laura MacNeill 00:55:23

I'll try that one. I think for CMT, it's a genetic disease, so there are genetic medicines that would be very specific, but it's also a neuropathy, which expands out the impact of our work into many other diseases: diabetic neuropathy, chemo-based neuropathy. So, I think that when you can focus on one disease group, and you've solved the delivery to the peripheral nerve, for example, you can have a much bigger impact on that technology from a financial return perspective. And so, I think it's our responsibility as patient advocacy groups to figure out what that big impact is and be able to bring that to industry. But all of us need to stop looking at it as disease by disease, specific by specific. We're just setting ourselves so far back. And so, if everyone could stop and zoom out at the bigger picture. I think you're saving individual lives when you're investing in a therapy, even if it's an N-of-1. But there's absolute learning that gets translated out to other diseases and the next project, and there's absolute ROI that's up on that. And the patient communities want to see industry lean into the rare diseases. I think that obviously that's what the big talk is. That's what we've been talking about this whole session, this whole week: that it's time for rare disease to get some attention. And so, when an industry will step up and support that, I think it's very goodwill showing to patient communities out there that are looking for it. And we know the math, right? There's plenty of people out there that have rare diseases. And so, think about the bigger picture, and I think it's our responsibility, too, as patient advocacy groups to lay that out for you very thoughtfully when we meet.

Sudhir Sivakumaran 00:57:01

The other point to that, if I quickly add. So, in—Lewy body dementia is not a rare disease, but we have the same challenges. So, the other way in which we can get the industry to come and work on the rare disease—she made a very important point. I wish she slowed down a bit because that was really important. The community right now, we tend to bind ourselves within these definitions of the disease based on the names we have given them, the clinical diagnosis. We need to go beyond clinical diagnosis to the underlying biology. Biology of a disease doesn't distinguish those names that we give to classify the patients into different diseases, and I'm sure this is the case in rare. Because from a drug development perspective, there is so much in terms of the core pathology, overlapping causes of the disease between many diseases, particularly in rare neurodegenerative diseases or even between rare and some Alzheimer's patients, right? So, if we can go beyond that, if patient organizations can say that, "We are, yes, of course, supporting one specific community, but we are in this to advance the field as a whole for the neurodegeneration," and that's going to be a critical piece. Move beyond the clinical diagnosis. Don't be

stuck on those clinical definitions of the names of the diseases we are given, because the biology doesn't see those names and boundaries. It spreads far beyond that.

Emily Taylor 00:58:20

Well said.

Ebony Dashiell-Aje 00:58:21

Wonderfully said.

Laura MacNeill 00:58:22

Well said.

Ebony Dashiell-Aje 00:58:23

Look at him. Rock star.

Ebony Dashiell-Aje 00:58:27

So, here's the last question from the audience that I want to give you guys. Do you have any tips on how best to approach industry? So, we talked a lot about how industry can be interested in patient organizations, but there's some proactivity as well that patient organizations can have to directly reach out to and start the conversations with specific industry partners. So, are there any tips on how best to find and also engage these partners?

Sudhir Sivakumaran 00:58:57

I can probably start because this has been my life for the last 10 years, and—I see my good friend sitting there; he knows very well what I mean. I've worked a lot in the industry. How do you get the industry to work? So, one of the things I would encourage the patient advocacy organizations, nonprofits, to do—when we go talk with the industry and ask them, "What is your need?" And then, "What do you want?" They're going to say a couple of lines. Read beyond that. I mean, not read between the lines, but think deeper about what they actually mean. Because when an industry is telling you about a specific need, we need to acknowledge that that's not a need driven by one variable, such as a scientific or a clinical program: there's a business need, commercialization need, market need, access need, a CEO's wishes that they may have that the unseen people don't have the luxury of knowing beyond. So, I think we need to create that value proposition to the industry that's strong enough to convince them to be attracted to what we are doing. That's the key. The value proposition is very nuanced. Talk about creating those real-world data, real-world evidence for biomarkers and endpoints. And get them to work with you. Even if we don't have something right now, even a theoretical white paper, that's going to get the industry excited because they're going to have a framework to refer to once they actually start doing something. So, creating that value proposition is going to be absolutely key, and we need to be patient with the industry. They are trying to say something, but they are sometimes laconic. Spend time thinking about what they actually mean and keep an open mind. That would be my best thing. And I've worked a lot with them, and I can tell you where I've been very successful thanks to the opportunities I've been given in terms of industry. And we've delivered on a couple of big projects purely with industry support, but we had to bring

them to the table first, and it's not difficult. Just be an open mind and give them the space that they can operate in.

Ebony Dashiell-Aje 01:00:47

Wonderful. Well, we're at time. Please join me in thanking our panel for today. Thank you all so much.

Disclaimer: This transcript was reviewed by individuals for accuracy and serves as a reference. However, it may still contain errors or omissions. Please verify any critical information independently.