Autumn Newsletter

Diane Gross, MPH
Lupus Research Alliance

Rodney Winley
CSL Behring

Julie Breneiser
Gorlin Syndrome Alliance

Hilary Wilson, PhD
Boehringer Ingelheim

Karlin Schroeder
Parkinson’s Foundation

Scott Gray
Clincierge

**Parkinson’s Foundation** on developing trials that matter to chronically ill patients

How **Boehringer Ingelheim** maintains accountability in incorporating patient feedback

**CSL Behring** shares how they disseminate patient insights across teams

**Plus:** Creating endpoints with rare disease patients, cultivating equitable patient partnerships, and increasing clinical trial diversity
Welcome to the *Determining the Impact of Patient Engagement* issue of the *Patients as Partners in Clinical Research* newsletter.

The interviews in this issue feature case studies and examples of patient engagement, endeavors to create concrete measurements of patient engagement impact, and the work being done to communicate value.

This issue features:

**Julie Breneiser**, Gorlin Syndrome Alliance, on how rare disease patient voices create endpoints that truly make an impact in clinical research design.

**Hilary Wilson, PhD**, Boehringer Ingelheim, on how Boehringer Ingelheim approaches patient engagement and incorporates patient feedback in trial design.

**Diane Gross, MPH**, Lupus Research Alliance, on how the Lupus Research Alliance cultivates equitable patient partnerships.

**Scott Gray**, Clincierge, on how patient engagement is the key to improving diversity in clinical trials.

**Rodney Winley, MBA**, CSL Behring, on leveraging the power of digital to interact with patients and share patient insights.

**Karlin Schroeder**, Parkinson’s Foundation, on working with pharma to develop trials that matter to chronically ill patients.

The Patients as Partners newsletter is the official publication of the Patients as Partners in Clinical Research conference, designed for R&D executives and patient advocacy. **Enjoy the Autumn 2021 issue.**
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Recommended Media

Enjoy more media of patient engagement in clinical research.

podcast 🎧 How to Utilize Technology to Truly Increase Diversity in Clinical Trials

podcast 🎧 Solving for the Patient Retention Problem in Clinical Research

podcast 🎧 Using Sensor Technology to Deploy Decentralized Trials and Improve Patient Care

webinar 🎬 Patient Data Access Initiative: Introduction to Patient Data Return

newsletter 📖 The “Patient Data Return” issue

newsletter 📖 The “Patient Centricity in Decentralized Clinical Trials” issue
How the Rare Disease Patient Voice Creates Endpoints That Truly Make an Impact

Julie Breneiser is Executive Director, Gorlin Syndrome Alliance. She became the Executive Director in 2019. Prior to taking on this role, she had been a member of the Board of Directors for several years and was the President of the Board when she moved to the role of Executive Director.

What is the work you’re leading at the Gorlin Syndrome Alliance?

We have a number of things going on at this point. As always, our first priority is providing support to individuals and families with Gorlin syndrome. The next big ticket item we have right now is on October 8, we’re doing an Externally Led Patient-Focused Drug Development (PFDD) meeting for the FDA.

This is really an opportunity of a lifetime for us to teach them about what Gorlin syndrome is, the burdens and the unmet needs, and what would make our lives better. We hope it will make a huge difference in drug development and the FDA’s recognition of our issues, our problems. We are also currently assisting in recruitment for clinical trials and preparing for 2022 when we hope more trials will begin. We’re working with pharmaceutical companies on that.

One of your pharma partnerships is with LEO Pharma. Can you describe that collaboration, and any other partnerships you’ve had?

One of the most burdensome manifestations of Gorlin syndrome is that we get multiple basal cell carcinomas. I’ve had over 1000 basal cell carcinomas removed. We were heavily involved with them, after the trial protocol was written, in assisting with recruiting patients. We had record-setting recruitment with that. We also assisted with retaining participants in the trial.

That trial unfortunately did not meet its endpoints. There were specific numbers, in terms of percentage response, that the trial needed to reach and it didn’t get there. That was hugely devastating for our community, because for some people, their response to the topical product was life changing.

They went from having skin cancer surgery on 20 or more lesions in a year to, over the course of two years, not needing any skin cancer surgery on their face, neck or scalp. That’s huge.

Is that type of feedback that you might pass along to the FDA?

It’s just part of what we hope will come out of our PFDD meeting, and all our communications because I feel strongly that there needs to be some regulatory flexibility when the FDA looks at rare diseases. Let’s say we had a product for hypertension, and had a response rate for a new product of 30%. That’s not very much; that’s not very good. But if in a rare disease, to have a 30% reduction in your number of skin cancers that you develop over a one-year period, that’s a big number for somebody like me.

So I think the recognition of the burdens as well as regulatory flexibility needs to be applied to all rare diseases, particularly ours in this situation, because it will make a positive impact on our lives, on every level. Using FDA lingo that I learned recently, it will be clinically meaningful in that there will be significant improvement in how we feel, function, and survive.

It’s a point that we have stressed with other companies, and we will continue to do so. Again, regulatory flexibility is essential, because those numbers are huge. The challenge is getting the FDA to approve a protocol with endpoints that are, in our minds, reasonable. And to me, reasonable is 30%. But in many cases, it must be 50% or higher. And, again, I go back to the example of a person that I know who went from having no basal cell surgery for over two years, while on this trial medication to being back to where he was before, where every three to four months, he’s having multiple basal cells removed.

I get that there’s also a financial component to it. But, this is not something that the FDA evaluates. How much the product is going to cost is another, obviously critical, component to our members.

By advocacy groups partnering with pharmaceutical companies, that helps pharma understand where we’re coming from, and what will be most beneficial for us.
One of the things I look at is the cost of having 30 basal cells removed per year, compared to this drug, or any drug. I am pretty confident that the drug would turn out to be less expensive than the multitude of surgeries required.

**How do you determine a successful and impactful pharma partnership?**

It has to start in the protocol development, because we need to be able to say, “These are numbers that you need to reach for in terms of endpoints.” We have data from a global survey that ended in early 2020 that shows our burden of disease, in terms of many manifestations, but particularly basal cells. We have data that shows the volume and can show what a percentage reduction would mean. It starts with protocol development, then designing a study to make it reasonable. We’re a community of people. Part of our global survey showed that individuals with Gorlin syndrome spend over 24 days per year either at appointments, surgery or recovery. They have missed that much of their life to all those things. So to add in a clinical trial, where you have to have multiple visits, may not be reasonable. So figuring out how to lower the number of in-person visits is critical.

Then it’s creating inclusion/exclusion criteria that are based on, for example, the number of basal cells that people develop. After that, there’s recruiting patients, retaining them in the trial and meeting with regulatory agencies like the FDA to clarify the positive impact of the product on our community.

**Why is it so crucial for pharma groups to work with patients or advocacy organizations?**

You do not know what life is like for somebody with a rare disease, particularly one that can affect every organ system. From our partnership with LEO came the phrase, “Nothing about you without you.” For any pharma company to say that they are sure something will make an impact, how do you know? Have you asked us? I feel strongly about “nothing about you without you.” For any pharma partnership? at one point say to me, “It’s just a basal cell, you can have them cut off.” And I said, “Wait a minute, wait a minute. What if you get 40 or 60 in a year? That’s not that easy.” Don’t make presumptions like that without knowing what it’s like to walk in someone’s shoes.

It’s not just that day of going to have them cut off. It’s the anticipation before. Then it’s the day of having them cut off. Then it’s the recovery which can take months. And if you are a parent, it is the time away from work to take your child to the appointments, the surgeries, and care for them while they recover. It’s how people look at you, how you feel about yourself, how you look, how you sleep. I had one removed from my ear three weeks ago, and I still can’t sleep on the right side of my head.

There are all these factors that come into this – physical and psychosocial and emotional – that need to be considered.

And by advocacy groups partnering with pharmaceutical companies, that helps everybody. It helps pharma understand where we’re coming from, and what will be most beneficial for us.

**Have you seen the change in the ways in which pharma companies are willing or accessing patient engagement groups or patient advocacy groups over your time as an advocate?**

Definitely, absolutely. They’re much more willing to have us as partners, and that’s really what it has to be for success in the long run. There’s no other way. I can only speak for Gorlin syndrome, and it also depends on the pharmaceutical company. So from my standpoint, the partnerships between advocacy and pharmaceutical companies are essential for trial success. We have a private and secure database; when, for example, when we have a trial that is recruiting, we promote that trial on our website, through all our social media, and at our conferences so that people are given the information on the trial and they make the choice as to whether they seek out more more information on the trial.

In my ideal world, and what is happening more and more, is that pharma is engaging with us to develop a win-win situation. If you’ve got a good molecule, you’ve got to develop a trial to highlight that product, otherwise, you might as well throw that money away. And we all know it’s a huge amount of money. So it’s essential to make it win-win as much as you can. When you have a population of potential participants who are hungry for better treatments, and hungry for a cure, why not go to the advocacy groups that will bring those people to you?

**Is there anything you’ve learned about how to partner more effectively with pharma?**

What always works best, in my view, is being open, honest and transparent throughout. You have to be careful, because of compliance rules. In the United States, and in Europe, there are certain things you can and can’t say. But one of the things that I’ve done all along has been to be open about saying, “Okay, I know there is a compliance box. Help me stay within the box and not jump outside of it and impair the integrity of the trial.”

**What would be your final message for any advocacy and pharma readers?**

For advocacy groups, I would say it’s critical to have data from a survey or “registry” to show your burden. With the Gorlin Syndrome Alliance, we used to say, “Basal cells are a huge burden.” From our global survey that I mentioned earlier, we now have the numbers, the data. So, do surveys to show the data, the burden, and the unmet need. It’s also the language of the FDA. They need to see the numbers. It can’t be subjective. It has to be objective: “I’ve had X number of basal cells on my face in the last 24 months. I’ve had Y number of basal cells on my trunk and legs,” etc. For pharma: use your resources. It’s silly to not do that, because you’ve got groups of people who are hungry for better treatments, and who can provide you with so much. ☚
How Boehringer Ingelheim Approaches Patient Engagement and Incorporating Patient Feedback in Trial Design

Hilary Wilson, PhD, is the Director of Patient Engagement in the Patient Affairs and Engagement Center of Excellence at Boehringer Ingelheim, supporting the advancement of patient engagement methods and approaches, and the evolution of the organization’s systematic approach to patient-focused drug development.

What is the work you’re leading at Boehringer Ingelheim?

I work in a center of excellence in the US organization focused on advancing patient engagement and our patient-centric culture. My role is primarily focused on patient engagement – which we define as the bidirectional interaction between the organization and our patient stakeholders to inform the design of our products and services. I work at a macro level across the company, optimizing our process and innovating the ways in which we engage with patients, as well as tracking and reporting on the patient engagement activities that the US is engaged in to our US human leadership teams.

How has the patient-centric culture at Boehringer Ingelheim specifically in tracking and applying patient-generated learning, evolved?

Our approach to measuring and reporting has evolved with the behavior that we’re trying to encourage. Initially, it was an activity-based metric: reporting to our US leadership team, “This is the frequency of patient engagement activities that were planned, and that had been completed.” The reason for that is because our leadership recognized the importance of patient engagement and wanted to encourage teams to adopt it more broadly.

They wanted to know the progress on that and if we were improving. But as patient engagement became more common and more teams were embracing it and doing it organically, we recognized that we needed to evolve and move beyond an activity-based metric.

And so we now capture additional information about the activities: What was the method used to collect the insights? What was the impact of the activity? On an annual basis, I do a qualitative analysis to understand what methods teams are using and how the insights are informing our decisions.

The behavior we’re trying to influence here is to encourage teams to use those insights in their decision-making, because our company believes that if we want to develop products and services that meet patient unmet needs, then we absolutely have to be collecting and actioning patient insights early, often and throughout.

How are you determining success in these activities?

We really look at success primarily based on two things: first, did the patient stakeholder(s) that participated in the engagement have a positive experience, and second, did the team consider those insights in their decision making? That’s not necessarily, “Did something that a patient said in the engagement result in a change?” Because if you’re doing patient engagement right and you’re considering patient perspectives from the beginning, you might not be changing anything. Because you’re hearing what they need, and you’re making decisions based on those insights.

It does mean, however, “Has the team talked about it and have they considered those insights in their decision-making?” Because what we have found is that sometimes insights are available too late, or even if those insights are available, they sat within one group, but they weren’t a part of the team discussion when they were actually making design decisions. Those are barriers that we’re trying to understand better, to then look at our processes and make sure that we’re solving for these challenges.

We have “patient minutes,” brief stories from or about patients that anchor the team in the conversation about what we’re trying to accomplish. It’s important to understand the disease areas that we’re working in, from that patient lens.
How are you engaging with patients?

We engage with patients in many different ways, at varying levels of engagement. At the most basic level, we have one-way patient insights: doing focus groups or interviews, even surveys, to understand specific unmet needs, preferences around outcomes, key symptoms and impacts, etc.

And then as you move up that ladder, you have higher levels of engagement. There is an advisory level, co-creation, and all the way up to being involved in patient stakeholder-led initiatives, where Boehringer Ingelheim is in a participatory role. All these different levels of engagement have a place.

How do you communicate the value of patient engagement activities back to the organization?

Because patient centricity is one of our guiding principles, I do think that value is understood. But how did we get here?

It’s really been a combination of factors. Part of it is more and more teams experiencing it for themselves. When you actually are involved in an engagement, you might go into that a little bit skeptical and think of it as a “checking the box”, but you’re going to come out of that exercise recognizing how important it is and understanding the value of gaining insights.

A big part of it is having funding resources, leadership support, and expectations that we will do this and having teams experience it for themselves.

But the other piece of it is also equally investing in the organizational culture. Within the US Center of Excellence, we also focus on supporting a patient-centric culture. So no matter where you sit in the organization you know, you have a patient-first mentality. One example is our patient ambassador programs, where people are nominated within their different functions and are trained on patient centricity and given resources that they can bring back into their teams.

Think of Starbucks: they start every meeting with a coffee tasting where the employees learn about where those beans are from. It’s just a part of how they start their meetings. We do it similarly: we have something called “patient minutes,” brief stories from or about patients that anchor the team in the conversation about what we’re trying to accomplish here.

Sometimes patient minutes are from someone from a therapeutic area team sharing the results of a patient engagement activity. “This is what we learned and this is how the team is using it.” Sometimes it is content pulled from the public domain.

For example, patients talking about the importance of diversity in clinical trials. Sometimes they are patients sharing their experiences living with a disease in a therapeutic area we are working in. It’s important for us to really understand the disease areas that we’re working in, from that patient lens.

Can you share an example of an outcome resulting from specific patient engagement activities?

This is an example that’s not tied to a specific insight, but about a way in which the team approached patient engagement systematically. We were working in a new indication for Boehringer Ingelheim. In preparation for the start of development – before we were even in humans – the Global Patient Advocacy team organized the first global patient advisory board meeting.

And in that meeting, one of the patient organizations presented results from a survey that really helped characterize the key symptoms and impacts and they described some of the challenges with existing clinical trial measures as they don’t align with the key symptoms and impacts from the patient perspective. And so because they were having that conversation so early, the clinical trial team was able to work with the patient community to include endpoints in our first clinical trial that were meaningful to patients. That’s co-creation.

What do you envision patient engagement in pharma looking like in 3-5 years?

I’d like to see it be systematic and seamless; it has to be built into the very way in which we approach everything we do across the lifecycle of medicines.

We’re certainly making great strides here at Boehringer Ingelheim in that area; many pharma companies are. Not any one pharma company does it all best, many shine in one area. Some excel in patient engagement in commercial, others in clinical development. But until we have patient engagement and patient insights seamlessly and systematically integrated throughout our process, we won’t truly be doing patient-focused drug development.

In terms of next steps: pharma has been doing a better job at getting patient input, but the next focus is going to be on representation in the insights that we’re gathering. It can’t be only with patient organizations that have a select patient population. We also need to be looking – within the United States – at the disease population, the prevalence and unique cultural differences and social determinants of health that impact the design of our trials or the way in which patients access and experience our products and services.

The same way that we’ve pulled in patient insights into our development approach, we need to also be pulling into these considerations very early and ensure that we’re getting representative input. It’s exciting to be a part of work that is rapidly advancing the science of patient input. ◆
Patient Engagement: The Key to Improving Diversity in Clinical Trials

Scott Gray is the co-founder and CEO of Clincierge, the leader in patient support services for clinical trials.

Since 2015, Clincierge patient coordinators have managed logistics and reimbursements for more than 140 clinical trials worldwide. For more information, visit www.clincierge.com.

According to a recent report from the FDA (United States Food and Drug Administration), in 2020, 75% of American patients who participated in clinical trials were white, with the remaining 25% made up of minority groups such as Blacks, Asians, Hispanics, and Latinos. However, according to the United States Census Bureau, members of these groups comprise more than 33% of the US population.

This sort of disparity in participation is not limited to clinical trials within the United States. Another study looked into 29 countries over the past 21 years and revealed that 86% of participants were white. This lack of representation is an enduring and complex challenge for pharmaceutical companies and clinical researchers across the globe.

Diversity impacts all industry stakeholders; pharmaceutical giants like Pfizer acknowledge that patients from different backgrounds respond differently to the same drugs and therapies. On a website dedicated to clinical trials, the company has publicly committed to reducing health disparities across populations and investing additional resources into drug research for historically underrepresented minority groups.

This significant step by Pfizer is one example of the industry’s renewed commitment to creating diverse, equitable, and inclusive trial practices.

Benefits of Patient Diversity

There are several reasons why it is in the best interest of both pharmaceutical companies and clinical researchers to recruit and support patients from historically underrepresented populations. With patient diversity comes an array of benefits that impact both individual participants in the trial, and the broader community of people with a specific disease.

Ethical
First and foremost, a lack of representation in clinical trials raises concerns about equitable access to cutting-edge therapies and quality healthcare.

In addition to pharmaceutical research’s scientific and commercial objectives, clinical trials present a critical lifeline for patients worldwide. Put simply, a person’s race, gender, age, socioeconomic status, or other demographic denominators should not prevent them from participating in a clinical trial. Each individual should feel confident a prescribed treatment will be safe and effective for them once it is available on the open market.

Scientific
Proportional representation helps clinical researchers identify when a new drug or treatment affects patients differently, as side effects and efficacy vary significantly across demographics. For example, it is well-documented that Black patients react differently to certain blood thinners and asthma medications when compared to other patient groups.

Demographically inclusive research can clarify and help address disparities before a drug manufacturer seeks regulatory approval. A clear understanding of the reaction of each demographic group to a new therapy is essential for the medical providers administering it to their patients in the future.

Commercial
Finally, in a prior issue of the Patients as Partners newsletter, I shared a related story that bears repeating. At a conference several years ago, I witnessed a remarkable exchange between two panelists. After a pharmaceutical speaker ended their thought, an FDA official boldly responded, “You’ve only tested your drug on 60-year-old bald white men; why don’t we approve your drug for 60-year-old bald white men and see if you’re able to recover your investment?”

The FDA has not been shy about the importance it places on patient diversity. In fact, in 2020, the organization released a separate report on diversity in clinical trial populations exploring eligibility criteria, enrollment practices, and trial design. In addition to the ethical and scientific benefits of patient diversity, these guidelines also increase the likelihood that new therapies will receive regulatory approval and result in commercial viability.
Make no mistake: patient diversity is critical to the success of clinical trials, but for far too long, the conversation has stalled at why. The time has come to start focusing on how. Understanding the circumstances that lead to underrepresentation is the first step to increasing patient diversity in clinical trials.

Overcoming Barriers to Participation

Recruitment and retention are among the most significant challenges facing clinical researchers, with more than half of trials delayed by low enrollment and some dropout rates exceeding 30%.

Trial participation often presents a heavy financial, emotional, and logistical burden for patients and caregivers, leading to recruitment challenges and poor retention rates. Truthfully, these sorts of hurdles impact nearly all patients somehow—but three factors seem to play a more significant role for historically underrepresented populations.

Awareness

Investing in education and outreach to promote awareness of clinical trials and the potential health benefits for trial participants – and the broader population - can go a long way toward attracting appropriate study participants.

Increasing site locations in minority neighborhoods and holding recruitment events during weekend and evening hours are simple but effective ways to make trials more accessible. Likewise, building relationships with local community leaders and healthcare providers also encourages the recruitment of underrepresented populations.

Finally, additional thought should be given to the design of trials to make participation less burdensome for participants. Asking patient advocacy groups and patients and caregivers themselves for input will help trial sponsors and researchers to avoid introducing trial components that inadvertently discourage patient enrollment and retention.

Economic Disparity

A recent study showed that patients in households making less than $50,000 per year are 27% less likely to participate in clinical trials.

In addition to communicating the potential health benefits of trial participation, trial sponsors and clinical researchers should take great care to address participants’ financial constraints and promote any financial programs that may be available to them. Informing underserved populations about compensation and travel reimbursements encourages participation; likewise, investing trial resources in increasing those funds helps address the disparity in incomes.

Additionally, prioritizing prepaid travel arrangements and ensuring rapid reimbursements for out-of-pocket expenses removes the burden from patients and caregivers worried about participation costs.

Those who recruit for clinical trials should also be prepared to discuss related third-party programs, such as the United States’ Ensuring Access Act, which deducts the first $2,000 a patient receives for clinical trial participation from federal benefit eligibility determinations.

Patient Uncertainty

Unfortunately, some populations mistrust the pharmaceutical industry due to past ethical breaches in diverse community. Researchers in the Tuskegee Syphilis study in 1932 withheld treatment from Black men to study the course of the disease. Almost a century later, black patients today are less likely than white patients to receive medical care for the same symptoms.

Recruiting diverse populations to participate in trials is only the first challenge. Participants must be supported and remain in the trial through completion to be represented in trial outcomes. For this reason, it is essential to promote the health and financial benefits of participating and provide direct support to participants throughout a clinical trial. This continuity offers reassurance and builds trust.

Partnering with a company that focuses on patient support creates a bridge between the patient and the clinical trial site. At Clincierge, patient coordinators are assigned to each trial participant for the duration of the trial. They assist patients and caregivers with managing travel, finances, and other logistics related to their visits. In other words, they alleviate the burden of participation for the participants and their support system. As an added bonus, they allow trial site coordinators to focus on other essential aspects of their studies, like data collection and analysis.

Coordinators often become a trusted “shoulder to cry on” for patients and caregivers facing the stress that comes with chronic illness. These relationships cannot be overlooked. While the average industry dropout rate hovers at 30%, Clincierge maintains a 95% retention rate among patients utilizing our services.

Reducing the logistical, financial, and psychological barriers to entry strengthens patient diversity and increases overall patient satisfaction with the entire trial experience. Investing energy and resources into patient logistical support will allow all of us to move research and medicine forward in a more equitable direction.

For more information, visit clincierge.com
How the Lupus Research Alliance Cultivates Equitable Patient Partnerships

Diane Gross, MPH, is Director of Public Health Information at Lupus Research Alliance. She leads the Lupus Research Alliance patient education and engagement programs and advocacy initiatives. Her work also focuses on bringing the patient voice into the clinical research and drug development process.

Can you describe the work you’re leading?

I work at the Lupus Research Alliance, which includes our clinical research-focused arm, Lupus Therapeutics. I’ve helped to develop and launch initiatives that are now being run out of the Lupus Therapeutics arm. One major initiative that I helped envision and launch is Patient Advocates for Lupus Studies (PALS), which is a peer to peer program being piloted at five academic medical centers that are part of our Lupus Clinical Investigators network, created and managed by Lupus Therapeutics.

People with lupus who have been through a clinical trial were trained to be peer educators – so that when the physician or the trial coordinators start to talk with a patient about possible involvement in a trial, they offer to put them in touch with a PAL as a resource to learn about trials from someone who has been through the process themselves. Another project I’ve helped design is the Community-Based Health Action Network to Generate Trial Participation and Eliminate Disparities (CHANGE project). That’s a three-year project to pilot initiatives in a few key markets to investigate potential programs we can offer in communities to raise the profile of lupus and break down the barriers to participation in clinical trials – whether it’s long-held perceptions, barriers of transportation or basic knowledge about clinical trials.

What is some of the work you’ve done to provide patient input on clinical trial protocols?

Patient Advisory Boards are some of the work we launched back in 2014, which was pioneering in this space at that time. We were approached by CISCRP to collaborate. They were working with a pharma company to get patient input on a clinical trial for lupus, and it was a real pleasure to work with them on this. Since then, we’ve done quite a few patient advisory boards, and it’s been nice to see the progression in how companies are taking this work more seriously.

When we look at the whole scheme of things, groups like CISCRP and CTTI have been instrumental in moving patient input into clinical trials forward and thinking about “measuring” the impact, which is difficult.

We have a lot of anecdotal responses, but not necessarily a lot of concrete data, and that’s still incredibly important.

Some of the important things that we’ve done and where we bring an added value is knowing the patients very well. And one of the things that the companies we’ve worked with have been very responsive to is the wide range of perspectives represented by the patients we bring to the patient advisory boards. There is a whole spectrum of patients: from the everyday patient to the professional patient. There’s value from getting input from that whole spectrum if you’re only going to people who are raising their hand saying, “Yes, I want to participate in this,” you might be getting a very skewed perspective.

We’re very cognizant of making sure that the patient advisory boards are representative, of who has the disease across various demographics, education and geography, as well as their involvement, engagement and familiarity in these types of areas. Because essentially, when you’re looking for people in clinical trials, they’re likely going to be people who haven’t participated before. That fresh perspective is important, as well as the perspective of people who may have participated before. That mix is also important. Something that we emphasize is moving away from this idea of a “focus group.” It’s a matter of semantics, but a focus group is a one-off activity.

But if you’re doing a patient advisory board, we set up the expectation that this is going to be ongoing.

Keeping people updated along the way, and getting their continued buy-in, is important.
Maybe there’s a major meeting in the beginning of the whole process, but keeping people updated along the way, and getting their continued buy-in, is important. It’s also about providing feedback back to the advisory board on how things are going. The first company that we worked with set such a phenomenal precedent in working with us on that, and had this very strong commitment to returning feedback.

Can you share some of the outcomes or impacts that patient engagement has had from your work?

I’ll share a couple examples of things advisory board members have raised in reviewing protocols and materials. Lupus is a disease that predominantly strikes women in their childbearing ages. And sometimes, repeated pregnancy tests are a bit of a mental strain. Pregnancy with lupus can be complicated, and you need to be thinking about it, planning for it and having it managed. And so for some people, the repeated testing can trigger a variety of feelings. Really think about, “Is it necessary every visit?” Another group, when they were shown certain literature, said, unanimously across the board, “I would never pick that up. That woman looks depressed.”

In a previous job, I was running a focus group for something unrelated, but our marketing team wanted to do a campaign. They had five mockups, and asked me to spend a few minutes reviewing the mockups and told me which one they thought was best. And the one that the team thought was the best was the one the patient group liked the least. They responded with: “You’re telling me this is a serious issue; show it to me. The message has to match.”

You’re expecting a person to basically put their life in your hands when they test a new medication. Obviously, they can reap the benefits, but there are a lot of risks. So doing advisory boards is incredibly important, and the volume of patient advisory boards that we’re starting to do now is representative of how people are starting to recognize the value.

What constitutes a good working relationship between pharma and patients?

There are a few things. One is to recognize that you have to be committed to patient engagement; it’s not just a checkbox. Because we will see that, and the patients we bring to the table will see that. So first, it’s important that you’re truly committed to this endeavor. Another is recognizing that this is a marathon, not a sprint. You should keep the group engaged throughout, because you will get much better input. A periodic check-in, even if it’s just a quick phone call, is appreciated by the advisory board members, because it says to them: “They didn’t want to just hear from me in the beginning. My input matters.” Follow-up is a very simple way for companies to show their investment, and their commitment to hearing the patient voice.

On the operational end, I’ve sometimes gotten contracts from companies that were designed for their clinical trial site, not a patient advisory board. I went back and said, “This has nothing to do with what we’re doing.”

That’s slowly evolving, and it may sound like it’s something that’s fixable, but companies have these huge legal departments while most patient groups like the Lupus Research Alliance are small. We don’t have those resources. It’s important to be thinking about that when you’re working with the patient advocacy group to make sure across the board that how you’re working with them is reflective of the type of work they do.

Another thing that we’ve found to be very effective on both sides is having a bidirectional relationship, and a single point of contact in both organizations. Pharmaceutical companies, even the smallest ones, usually are are much bigger than the advocacy groups they’re working with. Sometimes people at the company may not necessarily know who’s working with who at the advocacy group. And even though we’re a small group, we may have different people working with a company on different things. So a single point of contact in both organizations who knows the bigger picture and makes sure everything flows is just helpful from an operational standpoint.

How do you determine the success or impact of patient-pharma collaborations?

We’ll see the impact of the patient advisory boards that we’ve been doing more as the trials progress, and whether or not they enroll on time and are on schedule. That is the more global vision of the patient advisory boards potential impact. There are groups looking at metrics on how to quantify the impact.

On the personal level, it’s great when the companies interact with the patients, and they get to see each other, and make that connection that we’re all people, and the companies aren’t just these big monoliths. That there are people who work there who are very compassionate and passionate about what they do, and do care about the patients. The patients get to see that side and experience that and start to get a different feel for companies. It’s a win on both sides. It does make a difference when you have those personal interactions. Some of the people who are working on a particular disease are doing it for a very personal reason, and so it is very important to them, and they are going to focus on it and not necessarily just run off and jump to another condition.

How would you like to see the patient-pharma relationship evolve in the next 3-5 years?

I’d like to see patient advisory boards become a standard part of every clinical trial, because they are incredibly insightful. Additionally, my career has been focused in health equity. I would like to see the momentum continue around decentralized clinical trials as well as considering how to make things easier for the patient to participate while giving the researchers and the companies the information they need to determine safety and effectiveness. I also would like to see that the participation within clinical trials are reflective of the real-world population. It’s resource-intensive to do that, but it’s more resource-intensive for a company to instead have to conduct a very specific trial in a very specific group and recruit for that to gain the data about their drug for that population.
Leveraging the Power of Digital to Interact with Patients and Share Patient Insights

Rodney Winley, MBA, is a Senior Director, R&D Patient Partnerships, R&D Strategic Operations for CSL Behring. As global head of R&D Patient Partnerships at CSL Behring, Mr Winley leads a team responsible for ensuring that stakeholders within R&D have access to the tools and resources needed to embed patient and caregiver focus in their daily activities.

Your title uses the term “patient partnerships,” rather than “patient engagement.” Can you expand on the distinction?

When you stop and think about it, we partner with patients, and we partner for patients. It’s working with patients and caregivers, and making sure that when we conduct our clinical trial and R&D activities, that we do it with the patients and caregivers in mind, with the perspectives and taking their considerations.

My job is to help our R&D teams across the globe in keeping the patients first in all that they do. That includes the thought process, culture and activities that we’re engaging in: making sure that we’re incorporating the patient voice, getting patient feedback and co-creating with patients.

We also are trying to make sure that we cultivate a culture where we work for patients, making sure that every CSL employee is able to relate their job to patients, and making sure they keep patients at the forefront of everything they do. That is everyone from the custodial staff, to the bench scientists, to the folks that are responsible for the clinical trials, to our marketing and commercial folks.

Partnering for patients is making sure that we’ve worked together internally to do everything we can share information, share best practices, and all that cross department reporting.

You presented on a digital patient engagement platform at last year’s Patients as Partners meeting. Can you expand on that and how you determined the impact of it on patients?

The whole idea was that there was a lot of information that you can see in secondary research and in other areas where you find that patients just want their lives to be easier. Technology makes lives easier, for the most part, as long as it’s easy to use. The adoption rate of technology has certainly increased substantially even over the last decade.

Patient engagement can get expensive. How do you provide the greatest value to as many patients as possible, and engage them in a positive way, cost-effectively? Technology helps you do that, and so that was the idea behind the platform. Being able to share information with patients, as they learn about learning about a clinical trial, as they go through the process of informed consent, what’s entailed in the clinical trial – we wanted a way to easily do that.

A digital platform makes that very easy to do. It also provides messaging, giving patients the opportunity to interact with other patients.

Those needs were at the forefront. And then when I started looking at the broader landscape, for example, what’s happening with electronic medical records. In that case, you can get appointment reminders, you can have a conversation with your physician or nurse, you can get your prescriptions; there is a lot of good information that’s already there. Why not leverage that for clinical trials? We also looked at healthcare providers overall, trying to understand their use of it.

I did a bit of research and found several studies; for one in particular, they had surveyed about 800 healthcare providers in the US and Europe, and roughly 94% of those either had already, or were planning to, put in place some type of electronic patient interaction tool. Those providers had seen that the majority of patients wanted a tool like that.

Based on that information, and what we’re trying to do similarly in clinical research, we saw that it was a good opportunity. We started asking advisory boards and focus groups of patients and healthcare providers, to confirm if it would be something of value.

It’s really important to share cross-department the great work being done, to focus on partnering with patients.
How do you capture and convey the value of patient partnership activities back to the organization to foster the need and capacity for patient co-creation?

We’re doing a variety of things. We have several different committees and networks that we have created internally. One of those is called the Care Network, which we launched this year. It’s essentially an internal social media network. Our folks internally are able to get into this network, share ideas, converse back and forth, comment on different things, etc.

We do innovation sessions as part of that network; we share patient best practices, and then we also get feedback. That information is fuel for some of the online conversations that take place after that, as part of that Care Network.

The other thing that we have developed is the Patient Focus Knowledge Center. It’s a repository for resources, case studies and how-tos. If you want to do an advisory board, or patient outreach, etc, we have people within the organization who have done this before. We use the Knowledge Center as a central place to store that information, and provide case studies that show people the benefit and the power of the activities that we’re doing.

Combined, it’s about centralizing that information in a place that it can be synthesized to share with everyone, but also providing people with an outlet for being able to share their stories.

Why do you believe it’s so crucial to have a place for these interactions and resources?

In any pharma organization or drug development company, you have folks who are doing research. For example, on the commercial or marketing side, there is a lot of research and interaction being done with patients to learn about the patient experience and journey. That information is still valid, and can be used in designing clinical trials. So it’s really important to share cross-department the great work being done, to focus on partnering with patients. Those ideas generated can be used in other departments.

And it’s not just for people doing a similar project; it’s beneficial for people doing different things as well. One of the challenges that I have is that there are a lot of things that can be brought from other industries in how you deal with patients. If you think about customer relationship management tools that are out there for almost every other industry handling sensitive consumer information.

There are tools and activities that have been done in those industries for the last decade that still have not been brought into the clinical trial arena. There’s no reason why you shouldn’t. Those things are there. So being able to learn from other departments, but also outside the organization, is crucial to me.

Is there a particular impact or outcome that patient partnerships have had on clinical development you can share?

CSL is doing work with sickle cell disease. Just to give a sense of the depth, breadth and importance of patient feedback, one of the things that we did was creating an augmented reality tool that would be used at the investigative sites to tell patients about the particular clinical trial. We wanted to create avatars that the patient would be able to choose between; the avatar would then walk them through what the clinical trial would be about. We thought it was a great idea and would be easy for patients.

We automatically assumed that patients wanted to have people who looked just like them to be that avatar. And so, we created some initial avatars, but we also put an advisory board together to ask patients feedback on this.

We found that this assumption was not the case at all. Instead, patients were more drawn to avatars that were at least semi-professional in appearance – not necessarily in a white coat or looking like an actual nurse or doctor; did not necessarily have to be a person of color – and looked like they knew what they were talking about, versus someone who looked just like them. And so because of that, we were able to create better avatars for them to choose from.

Even on that level, where you wouldn’t think patient input was as viable, it played a big part in patients being willing to and feeling comfortable with using the tool. Another example was around payment. A lot of people think that people are in clinical trials for monetary remuneration. I’ve conducted many patient advisory boards and focus groups, and one of the questions we put in front of our advisory boards was, “If we are providing a stipend, or paying back a patient, what would that look like?”

And interestingly enough, people were saying, “If you can contribute to a cause for people like me, that would be important.” They were more concerned about trying to contribute to the greater cause, then they were about trying to make sure that they got money for clinical trials.

What would you like to see patient engagement look like in the next 3-5 years?

A big thing for me is the decentralization of clinical trials. If you have 30 visits that are going to take place in a clinical trial, and 15 of those are taking samples, do you have to drive 40 minutes to that doctor site to do that? Can you do it somewhere closer or at home? What is the role of technology in making it easier for patients to submit or share information, and meet with their doctors? How are we leveraging telemedicine to make participation easier? How are we decentralizing trials to put patients more in control of their fate when it comes to trial participation? There’s so much opportunity for patient engagement to understand exactly how best it would work, what people are willing to do versus not willing to do, what they’re comfortable with, etc.
Working with Pharma to Develop Trials That Matter to Chronically Ill Patients

Karlin Schroeder is Associate Vice President of Community Engagement at Parkinson’s Foundation, where she leads the Parkinson’s Advocates in Research program (PAIR). Ms. Schroeder creates and directs projects to incorporate patient expertise into research with industry, academic centers and government.

In your work, what is impactful patient research engagement? And how do you measure that?

We always debrief after a patient engagement project, where we talk with people with Parkinson’s and the company about how things went. A successful engagement, from what we hear, is where everybody is saying, “I really felt like my voice was heard; I had lots of opportunities to speak up.” And where the company is saying, “We couldn’t believe we heard this insight; it just opened our mind to the fact that we were thinking about this part of the study in a really different way.” Another indicator of success is when we all want to work together again. This tells me that everybody felt that the interaction was positive and felt supportive. And that we are creating positive changes in the research.

In terms of measurement, we have looked at guidances such as the Patient Focused Medicines Development’s quality guidance. This guidance has seven focus areas that support planning for aspects of patient engagement such as ensuring everyone, researchers and patients, have shared purpose and have been prepared to work together and that a representation group is brought to the table.

We looked at all these tools and resources and came up with our own proposed metric set that could be used across disease areas, because at the time our landscape review did not find a universal set and we felt that was needed to compare metrics across the field. We send this to both the researchers and the patients involved with the project – and ask questions such as: Was the quality of patient engagement good? Were you prepared? Do you as a patient feel like you gave meaningful impact and were heard? Do you as the researcher feel like you’ve got meaningful information in?

We have initial metrics on the impact of patient engagement but there are challenges. It’s one more thing for people to complete. And for patient advocacy groups, often with a small team, it can be hard to constantly incorporate this step.

However, we are committed to advancing our metrics collection so that we can pinpoint at what stage of research patient engagement made an impact and what quantitative change happened because of that engagement.

Can you provide examples of how Parkinson’s Foundation used patient engagement to make trials more relevant to the patient experience?

Five years ago, the Parkinson’s Foundation partnered with a drug company doing two different studies with a similar study drug, and they were having trouble with recruitment. We led a patient advisory board for one of the studies to look at the protocol and determine what protocol amendments can be made from the community perspective, to make that study easier to participate in. Through that process, we were able to look at all of the secondary outcomes in the study, and say, “This may be too many secondary outcomes. Can we narrow them down to the two that are most important to the community?”

We were able to successfully reshape the study by reducing the burden of the study, including having more home visits and having less secondary outcomes, therefore fewer tests for patients. The anecdotal feedback we got from the company was that the study that had patient engagement led to faster recruitment. The study that didn’t have patient engagement was still struggling.

Another success story is having patients as partners on an international research team with a company to develop new patient-reported outcome tools. This project included asking questions as simple as, “Does this language make sense to the community?”

And you might think with England and the US that you’re not going to have that many differences.

If you really want to do meaningful patient engagement, you need to have a group of patients you can talk to about target product profiles, and the assets you may wish to acquire, and start from there.
But in the US, we don’t say, “Do a zip.” You “zip your pants” or “do up a zipper.” It seems like a small thing, but it ensured that the language was aligned with the community. And even in the answer keys of “On a scale from 1 to 5, how difficult is it to do XYZ?” the wording matters. Are we measuring difficulty? Are we measuring how important this thing is to us? Or are we measuring that, because it’s difficult I need help, or because it’s difficult I have to do it slower?

And lastly, we’re working with a company right now who’s supporting our work to reach under-engaged communities. We’re trying to reach communities of Spanish speakers. We recognize everyone has different language preferences and abilities, which we strongly believe should be accommodated taking the approach of health equity and inclusive research and care.

For example, bilingual Spanish-speaking people may prefer information in their native language. Or, migrant seasonal farm workers and older first generation immigrants might be monolingual. Literature and best practices show that language can be a systemic barrier to engagement in research and care. We support the right for people to have access to information in the language they prefer and the critical need for institutions to create that access.

With this project, we are training “promotores,” who are community health workers who can speak in Spanish, about Parkinson’s disease. We created an all-Spanish language training to prepare the promotores to talk about a variety of topics: “How do I identify if I or a friend might have Parkinson’s? Where do I find the care? And how do I address this situation as a care partner? And then how do we talk about research in our communities, particularly genetics research?”

What I loved about this partnership was that the company understood that we can’t just go into these new communities and say, “Take part in research,” when their communities experienced systemic racism in research. They were not saying, “You have to recruit for our studies,” but instead they were saying, “Let’s develop trust and an understanding of the community.”

Our evaluation of the program found that we were able to change the attitudes of the promotores towards genetic research. By the end of our work together, they felt that genetic research was important for Spanish-speaking communities and they should be talking about it.

What do you think are the need-to-have’s of working effectively with patients and pharma?
We have learned that it’s important to have a dedicated person who can really focus on these partnerships, because they are complicated, both for the patient groups and pharma partners. We are working in international and national coalitions to create a paradigm shift where the time needed for patient engagement is more frequently built into development timelines.

The current short timelines require an ability to work more quickly than is often times realistic for either partner.

It’s important for patient groups to understand the process that industry teams have to go through, and then have somebody with the knowledge and skill set to navigate that process. And on the pharma side, it is important to understand that patient advocacy groups are not the same partners as your typical third-party vendor. So it’s going to take a different approach to how contracts look and a different level of flexibility. It is important to ensure that everyone engaged in the work understands what patient engagement is, why it’s important to the work, and what might be different about that approach than your approach with other partners.

And if you’re new to the space, be open to asking your partners about what resources exist and how to get started. We have found that new partners are often surprised to find out that there are matrices to figure out how to do a patient engagement project and that there are planning tools that exist. It can still feel to some people a little amorphous but there is a whole science of patient engagement: there are methods, there are techniques, there really is a whole field to support industry teams.

What would you like to see patient engagement look like in the next 3-5 years?
Our team at the Parkinson’s Foundation is on the track to one way of how the future of patient engagement could look. We’re working with a partner right now to create a patient council that’s embedded in the company. This is happening in a few other spaces as well. This is the future. If you really want to do meaningful patient engagement, you need to have a group of patients you can talk to about target product profiles, and the assets you may wish to acquire, and start from there.

And once you have the assets, think about what the clinical development process is going to look like. And then you get into those details of designing study protocols, and those components. So it’s about embedding patient engagement to ensure there’s no one-and-done. It’s a continuous process and it allows you to do it early and often. It also exposes your whole team to patient engagement, where it just becomes part of what you do.

And the other piece of this is the concept that patient engagement becomes systemic and institutionalized. I worked on the Clinical Trials Transformation Initiative’s Quality by Design project, which has this concept of creating trials, where you no longer have errors that matter. You’re creating a safe trial, but you’re doing that through good quality study design with all stakeholders involved. And this work was actually embedded into the International Council of Harmonization provisions, saying that you need to do patient engagement in your work if you truly want to have a well-designed study.

There can be a drawback to rules and regulations dictating what patient engagement is and when it happens, in that it makes us lose some of our flexibility and creativity. However, it’s important to have these things overtly stated in regulations to ensure that patient engagement is just part of the process and is systematized in the work.
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