INCREASING PATIENT PARTICIPATION IN CLINICAL TRIALS

PANEL 4

Patient-Friendly Studies

One of a series of live panels from the WCG Patient Advocacy Forum in Washington D.C. in October, 2019

Introduction

How do we make clinical studies more patient-centric? What does that term even mean? In this panel discussion at WCG's Fall 2019 Patient Advocacy Forum, Steven Taylor, president and CEO of the Sjogren's Foundation and Ellen Wagner of Parent Project Muscular Dystrophy (PPMD)— and the parent of a son with Duchenne Muscular Dystrophy—take on these questions. Together they offer insights into how patients and advocates can amplify their voices and provide meaningful input into clinical trials.

These discussions have been significantly edited for clarity and length.

The Panel

MODERATOR

Danya Kaye

Director of Business Development
R&D and Innovation, Inspire

PARTICIPANTS

Steven Taylor

President & CEO, Sjogren's Foundation

Ellen Wagner

Founding President & CEO of Parent Project
Muscular Dystrophy

The Forum

Danya Kaye

"Patient centricity" has been a buzz word in the clinical trials industry for a while, but really there's no consistency in terms of what it means to pharmaceutical and biotech organizations. What does it mean to be patient-centric? Is there a better term?

Ellen Wagner

At Parent Project Muscular Dystrophy, instead of patient centricity, we typically say, "patient-focused drug development." Ours is primarily a pediatric disease: You're serving two audiences, so you have to keep that in mind when you say "patient-focused." "Patient" includes the caregiver or the family.

Steven Taylor

"Patient-focused drug development" keeps the focus on the fact the drug is for the patient. I think we should know that is the most important thing anyone can take away from it. It involves making sure the key players are in the room, not just the patient advocacy staff. The scientists, the clinical monitors, etc. are all part of that process, so they can all understand the disease as they look at the drug, molecule, or compound they're investigating.

Danya Kaye

What do you both see as the biggest challenge in being able to provide patient input effectively from your organizations to the industry?

"You're serving two audiences, so you have to keep that in mind when you say "patient-focused." "Patient" includes the caregiver or the family."

—ELLEN WAGNER

Ellen Wagner

I think one of the challenges is the squeaky wheel. The squeaky wheel is available to give input, and we have a great patient base that we get input from on a regular basis. We run focus groups with industry at our annual conference that are very well attended, but the only patients able to attend are those who have the means to get there. So the biggest challenges are, how you find the family in Tennessee with two Duchenne-disabled boys and how you make sure we're getting their voices heard.

Steven Taylor

I think it's so important that you look at who you're putting in the room or in the focus groups to ensure representation. Our most successful engagement process was with a major biopharma company. Before the protocol was finalized, they interviewed about 40 patients and chose nine that represented the various subsets of our disease in terms of age, disability, geography, symptoms, and other factors.

Everyone came from the company with a humble heart and open mind to learn from our patient population, understand the disease, and really listen about how it affects patients. And the moderators made sure everybody got to talk. Sometimes the squeaky wheel talks a lot and the others just nod. Everyone thinks they're in agreement, but they're just nodding because they're shy. So you need to call them out and make sure they get a chance to speak as well. I think that's very important, so you don't get just one perspective and think it's everyone's.

"Sometimes the squeaky wheel talks a lot and the others just nod. Everyone thinks they're in agreement, but they're just nodding because they're shy. So you need to call them out and make sure they get a chance to speak as well."

—STEVEN TAYLOR

The other challenge with patient involvement is we believe it should be very long term. It shouldn't be one and done. So it shouldn't just be a focus group that you've done, you've checked your box, you've interviewed patients, you think you know the disease and on you go. It's important to involve patients throughout the clinical trial, especially if you can start before the trial, but we know many companies just don't do that.

I think long-term involvement is where the challenge is; companies aren't always set up to do that, and you might not have the buy-in from everyone in the company. Having the patient advocacy or the patient centricity person run the program is great, but researchers and the company's higher-level people need to be there too because they're the ones that really need to hear the stories and learn about the impact of the disease.

Danya Kaye

And I think that's a really good point because as a vendor that serves the pharmaceutical industry, getting things like patient feedback on protocol design, a lot of sponsor companies come to us because it's a check mark they need to be able to make. They have patient and caregiver feedback, but they're actually not building in enough time to take action on that data. Their protocols are already in the process of being finalized. They're getting the feedback just to get the feedback.

A lot of organizations have their patient engagement groups, but those groups don't always own the budget to be able to take on these

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—DANYA KAYE

initiatives. And that is a huge, huge challenge. I speak to very well-intentioned patient centricity groups and patient engagement groups across many pharma and biotech organizations; the intent to gather the patient feedback is there, but they're having to convince internal study teams to allot part of their budget toward these efforts and convince them it's going to translate to better enrollment, engagement, and retention down the road.

These organizations need to streamline the process and make patient engagement a part of the entire clinical development operational process.

Ellen Wagner

One of the things we've done in Parent Project Muscular Dystrophy is patient-focused surveys. We have really good data on patient populations, so when a company comes and wants to design a trial protocol on this disease area, usually their first stop is our office.

I think there's an advantage in rare disease because recruitment's really tough. So they have to figure out how they're going to recruit patients. That makes an organization like ours more important to them because of our access to the patients.

Danya Kaye

How do patients and caregivers learn to give actionable feedback to sponsors? What advice would you have for somebody starting out who might be trying to really get that feedback back to the pharma companies?

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Ellen Wagner

Again, I think my experience is a little bit different in rare disease. In the instance of my son's trial, it was the first drug to go to trial in the disease space. He was one of the first 12 boys. The CEO and I know each other on a first name basis and have for a lot of years now. So the feedback loop was very close.

Then to the credit of the sponsors who ran the next study, they've looked very closely at that first trial, what worked and what didn't, and they have adjusted the protocol to make sure it was successful in their first trial. There were a lot of bumps the first time, like there always are, but I think because the feedback loop was so tight and there was some continuous improvement, the whole space has benefited from continuous improvement. But again, that's for rare disease. I don't know whether it's applicable in the larger environment.

Steven Taylor

Data is always important for pharmaceutical companies. Surveys and other successful ways to collect data from your patient population is really important.

If you're going in as an individual patient you need to have data about your own disease - not just how you live with it and what it costs to live with it, but what it's like to live with it. And then the story needs to be relatable and concise enough to make someone understand it. When you get patients in the room, they're eager to tell their story, but we need to make sure they are telling the story in the right way. Educate people,

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don't lose them after a few sentences, and don't go down rabbit holes, telling them about something at the pharmacy that happened last week week because they didn't have a thing on aisle five. That doesn't have anything to do with your story of living with the disease.

We encourage patients to write their story and tell it in a helpful way. I always tell them, put yourselves in the shoes of the people in the room. What do you think they would want to hear if they're a researcher, a clinical investigator, and what do you think is going to help them do their job? That's what they need to hear from you. Come in with that perspective and you'll be a lot more successful as a patient advocate.

Ellen Wagner

And to add to Steve's point, data is huge. When I entered the space in Duchenne, we didn't have any data. One of the first things PPMD did was set up the first registry for Duchenne patients. We're on our second iteration now. A few other countries have partnered with us and use the same concept we've put together, but that data is invaluable as pharma comes into this space to look at what they might be able to do for this disease. If you're a new advocacy group, is there a registry? And if there isn't, how do you put one together?

Danya Kaye

How have your organizations used social media to collect feedback from the community?

"...what do you think they would want to hear if they're a researcher, a clinical investigator, what do you think is going to help them do their job?"

—STEVEN TAYLOR

Ellen Wagner

We don't formally collect feedback through social media, but we definitely monitor social media closely. Many of the social groups we're aware of are private or closed, so the sponsor organizations can't monitor those groups or be involved in them, and they shouldn't be. It should be a place that parents can talk freely, but if we see something in our organization that looks concerning, we will always reach out to our sponsor partners and say, "We're reading this. You might want to take a look at education in this area," or "This clinical site is a concern. You might want to reach out." One of the things we've found is that the more clinical sites there are available, the more variability there is between sites, and so because of social media, parents are really tuned into each other.

Steven Taylor

We do a lot of social media pushes related to educating our patient population around what's really involved in a clinical trial and how beneficial they can be to the patient. They might get better care than they were getting before entering that trial. We tell them they aren't going to be guinea pigs, etc. That's a big myth out there.

We also started our own clinical trials page that lists the clinical trials currently running or actively recruiting. Clinicaltrials.gov is not updated; it's outdated and doesn't make sense to people. We post anything we know about the trial, including the IRB-approved flyers, so patients can find every trial in their area and call to learn more. We take down the information when the trial is full or closed. They often don't do that at clinicaltrials.gov.

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Ellen Wagner

We do webinars on a regular basis, and if we see a trial coming up on Duchenne's, we'll do a webinar on it. We're doing a series on gene therapy. That's very big right now in our population, but that's a really hard concept to get your head around. We're breaking it down and going through the steps. We've just started offering webinars in Spanish. All the webinars are archived, so they can be accessed anytime.

We also have trials up on our website. We do blogs. Anytime a press release comes out that's in our disease space, someone from our organization will do a blog and explain what it says and post the release as well. Sometimes the releases are really technical, so we try to break that information down.

Steven Taylor

While we're on social media, I can't tell you how many times we've seen this happen: a company will hire a third-party vendor to learn about the disease; the vendor does some landscape survey, reads some Facebook posts and some other social media, then think they know the disease, without ever having talked to a patient.

Then they will say, "Well, we interviewed 20 patients worldwide." I mean, literally. And we're like, "Twenty patients worldwide? We have a survey that we just did with 3,000 patients using Harris Interactive."

Danya Kaye

There's a lot of work that needs to be done by the industry to work

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more effectively with patient communities to really help reduce the participant or patient burden. How do you think the industry can better work with patient advocacy organizations and that process?

Steven Taylor

Bringing in Patient Advocacy Groups as early as possible—before the trials are designed—is the key to really making sure you're not trying to fit a square peg into a round hole. It is so important we get them involved early on and all the way through the process.

Ellen Wagner

We have partnered closely with the pharma companies in our space. Generally, they reached out to us. Back in the beginning, before we had so many clinical trials, we really started our relationships at the clinician level, then moved into the pharma level naturally because we were already involved with the clinicians and had built a reputation among them that were reliable. They were interested in doing the best thing. The relationships with pharma have just continued from there.

One thing we did was set up a certified network of care centers. Two or three centers that were garnering most of the patients, but not everybody can travel. So we started to look at how to arrange clinical sites more geographically. We have a steering committee and criteria that clinics need to meet. We review the criteria on a regular basis and certify clinics.

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Steven Taylor

For site selection, for example, for the Sjögren's Foundation, we have

76 sites we recommend in the United States and Europe for biopharma companies to look at to do clinical trials. We know they have the patient population, the interest in and the ability to do clinical trials, both academic and community-based rheumatologists.

I think a critical step in site selection is making sure we're working with companies to identify which ones have the patient population. Similar to PPMD, we also started with the clinicians. We didn't go right to the company. We went out and put together key opinion leaders who introduced us to companies they knew might have an interest. And from there, we built numerous in-depth relationships with every company because one person in a company can kill an entire pipeline.

You need champions at the pharmaceutical company. The clinicians introduce us, and we build on that. We do that across companies.

At one point, we had all our eggs in one basket with one major pharma company, and unfortunately, a new CEO came in. They cut the immune system pipeline; Sjögren's got cut. We were in clinical trials, but they cut the whole thing without even looking at the data. We thought this was going to be our breakthrough for a drug for Sjögren's.

What we learned was we had to cast our net wider. We went to every company we could go to. Now we work with nine. We built the depth at every company. We were able to do that thanks to the introduction of the clinicians and the partnership with the clinicians: they bring credibility to the table. If we can bring a rheumatologist into the meeting

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or scientists, then that really helps as well.

Danya Kaye

Can you talk a little more about how you're helping patients navigate the clinical trial process and what that means for your organizations?

Ellen Wagner

We do a lot of outreach, and we do a lot of education. Much of it is social media-based because that's where our communities come together. We hold an annual conference and talk about trials and opportunities for trials there. We hold regional conferences. We provide genetic testing if your insurance doesn't cover it or if you're looking for carrier testing and it's not covered. Most of our treatments now are genetics-specific; there are different gene errors that cause Duchenne's. You need to know exactly what genetic error is causing the disease, so it's important you get that genetic testing done.

Our registry reaches out proactively. Every time a trial opens, our registry scans for anyone who might be eligible for it, then it sends emails directly to patients. We send a set number of emails explaining that you're eligible. Please contact this number so you can call our registry and speak to our genetic counselors. They can talk people through the options of the trial, as well as give them whatever the local clinical site, the closest site, would be. And more information about the trial.

Steven Taylor

Obviously webcasts, etc., as well as our national conference speaking

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about the benefits of clinical trials. I always tell patients to call to learn more about any clinical trial. From there, they'll get a mailing from us, even if they don't qualify. A call doesn't mean you're signing up. It's great for the investigator or the coordinator to be able to talk to you on site, and once again, it reinforces the concept of education we've been talking about.

This may be a little off track, but I want to give a specific example that may help paint the picture here. We've learned that investigator locations sometimes have the worst customer service, obviously a turn-off for patients who are new—or even experienced—to the clinical trial space.

A company contracts with us to help recruit for their trial. We have a database of over 100,000 patients. But before we do a mailing or an email campaign to Atlanta or Philadelphia or wherever the site is–within a 60-90 minute drive–we'll call the office ourselves, pretending to be a patient. This is to see how they answer the phone. If it goes to voicemail, we want to see if the voicemail identifies who it's going to. Often, it will be something like, "Hey, it's Mary. I'll call you back when I get a chance. Thanks." And you don't know. Then you wonder if you dialed the right number. "That didn't sound like Doctor Livingston's office. That's weird."

So we encourage them to change their voicemail messages. When a person hangs up, they're not going to call back again. Everybody who calls is a potential candidate to be in a trial. We have nine companies doing trials. We can't turn people away simply because Mary has a weird voicemail or it's full, which happens all the time as well. We also

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call the front desk at the site to make sure whoever answers the phone knows there's a clinical trial happening for Sjögren's.

You'll see great practices that have great staff and are very successful. And we'll see some others that aren't so great. Sometimes it's the person who happens to answer the phone. Nothing to do with the doctor/investigator at all.

Danya Kaye

Ellen, in your personal experience as a caregiver for somebody in a clinical trial, did you feel you were an active participant in the process? And more broadly, how do you feel sponsors can engage with caregivers more effectively?

Ellen Wagner

I'm speaking from a pediatric perspective as the parent of a child in a trial. I am the person the sponsors are engaging with. They don't want to ask the six-year-old's opinion. In fact, they try to avoid asking the six-year-old's opinion. They're much more interested in what the parents are doing, and they're really interested in the parents being compliant. If you're not compliant, it really skews the trial.

One of the outcome measures that is very commonly used in Duchenne Muscular Dystrophy is the six-minute walk test.

Duchenne is a muscle-weakening disease that's progressive; it's very hard to show improvement in a progressive disease. What is

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improvement? Stabilization? In many ways it is. In many ways it's unfair to expect to see improvement, but stabilization means the drug is working, but you must show the FDA efficacy. That's a complicated task. The six-minute walk test was already approved by the FDA for other diseases. We can show whether the kids are walking faster or slower.

I don't know if any of you have small boys, but small boys are anything but compliant. You're three minutes into the six-minute walk test and the shoelace isn't right. They're going to stop to tie their shoe. Okay, that's the end of that test. Or you're in the hallway in the hospital and somebody opens the door from their office and walks into the hallway. Now the kids are having a conversation. That's the end of the six-minute walk test. Or there's a new picture on the wall, and he stops to see that picture. All these things happen on a regular basis.

The more the caregiver is involved, the more the parent is involved, and the more you can see what can happen prior to the clinician seeing it. Like I can see that the picture's moved. So before we start the test, we're going to talk about Winnie the Pooh, so that's off the table before we start. I think that when sponsors stop and listen to what the parents are saying about what they can expect from a little boy, the trial design becomes much clearer and much cleaner.

Danya Kaye

What about in Sjögren's?

"I think that when sponsors stop and listen to what the parents are saying about what they can expect from a little boy, the trial design becomes much clearer and much cleaner."

—ELLEN WAGNER

Steven Taylor

Caregivers can tell the real story and maybe validate what the patients are saying. My mom was a Sjögren's patient. She will tell you one story of what it is like to have the disease because she's learned to kind of hide the disease. But she's in bed at 7:00 at night because the fatigue is so exhausting. Or on the weekends, sometimes the joint pain is so bad that she can't really get out of bed, but she won't tell that story. The caregiver can tell the full story.

There's also the doctor. But it is a challenge: Doctors are sometimes misleading the pharmaceutical company. Doctors don't always know what patients want or what patients are willing to do. Now, I think doctors are amazing, and they don't mean to mislead, but you have to put together the doctors, the caregivers, and the patients' stories to get a complete picture of a disease.

Danya Kaye

Based on your experience, what sort of action-based solutions or lessons learned can you impart in terms of what is working in order to more effectively make studies patient-friendly by incorporating the patient voice in drug development?

Ellen Wagner

One of the areas we still struggle with are the IRBs and the difference between IRBs from organization to organization, from clinic to clinic. As a patient, I think it's really complicated when you're ready to start and the IRB doesn't approve for whatever reason. Or, one site is up and

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running and another is eight months behind, and that eight-month delay is solely because the IRB hasn't made the approval yet. Standardizing IRBs is one area we can continue to improve in terms of the patient voice being heard. I don't think the patient voice is heard at all in the IRB process. Clinicians I have talked to about this say the clinician voice is not always heard at the IRB-level either. We can start to look at IRBs and incorporate patient focus.

Steven Taylor

First of all, I'm a big proponent of pharmaceutical companies (not just in the clinical trial space) educating their doctors. They are amazing. We're trying to help them do what they do. But what we ask from them is to come in with a humble heart and an open mind.

If a clinical trial is already set-in-stone, the patients don't understand why they're being consulted. But if you bring patients in early enough, you could make changes based upon their feedback. They understand living with a disease. And if pharmaceutical companies come in with a humble heart and an open mind, they will hear the real story about the disease. They'll hear the roadblocks for entering a clinical trial. They'll hear the roadblocks of living through a clinical trial. And I think the lesson learned is this: Make sure as many people in the company as possible attend meetings with the patients. That will help every part of the process.

Danya Kaye

Embedding that culture of patient centricity within an organization

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—STEVEN TAYLOR

is sometimes really challenging. I am personally a cancer survivor. A clinical trial saved my life with a drug called Adcetris. I was invited to speak at Seattle Genetics—which produces the drug—about my experience. I addressed about 300 people across the organization. I had countless people come up to me saying, "This makes my paperwork so much more meaningful. I've never heard from a patient, and I've been at this company for seven years. I'd never heard how the work I do on a day-to-day basis makes a difference."

Audience Member

How do you manage the expectations of the community when clinical trial development can last up to 10, 15 years?

Ellen Wagner

You know, that's a continual challenge in our disease. We don't ever use the word "cure." It's a systemic disease you're born with, and so how will you ever cure it? We're hopeful for better treatments, for maintenance treatment, for less disabling results, and ultimately, not ending in fatality like it does right now. But that is absolutely something we struggle with all the time. We do a lot of work to educate people's expectations of the drugs that are currently available.

And the other part—that we haven't really talked about at all today—is how to pay for the drugs once they get to market. We have a drug that's somewhere around a million dollars a year. Now, if you need to combine that with another drug, who's going to pay for that? Gene therapy is even more. And in Duchenne, because muscles regenerate,

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—ELLEN WAGNER

it's probably going to need to be re-administered. We do a lot of work in educating our patients to set realistic expectations because that's a huge challenge.

Steven Taylor

Our disease is not always life-threatening. It's more life-altering and potentially disabling. But we don't use the word "cure." We use "conquer." So our mission/vision statement is "to conquer the complexities of Sjogren's."

We use clinical trials to understand how we're conquering the disease because everything we learn from clinical trials is helping us know more about the disease, among many other things we're doing to conquer it as well. Conquering is our key word, not cure at this point.

Audience Member

How, as a vendor, do we help convince organizations to invest in these patient engagement efforts (education of investigators, direct communication with patients and Patient Advocacy Groups, etc.)?

Danya Kaye

There's a couple of ways we've been able to do that. A lot of it has been through case studies - showing the impact of the data we collected. Showing where the key points in patient burden are, the actions taken to reduce the patient burden, and demonstrating tangible outcomes that have been helpful.

I think in terms of social listening or social media research, it's being able to show where patients are going at this moment in time to find information about clinical trials. What are the discussions and perceptions around clinical trials, including competitive trials? Who are the top bloggers and influencers in a particular space? If you're working with your sites, they will likely be able to tell you how many patients they think they have in their pool. Or you can look at the sites that have successfully enrolled in that particular therapeutic area before and understand who the online influencers are on Facebook, on Twitter, and on different health forums in different countries. Which are the leading patient advocacy groups that are communicating globally? How do you better engage with them? Some organizations have a strong presence on Facebook. Some have an annual event that's important to attend.

Truly, market research is not an expensive effort, and that's kind of what I go back to. So if you're shaving off a week of your enrollment time based on investing $50,000 on market research up front, then that is worth it, you know. That is going to be worth it for the drug development for the patients you're looking to serve, and it should lead to better retention in the study as well.

I think case studies are a great way to do it. So are examples of other companies that have successfully incorporated feedback that we've been able to provide to them in order to reduce their patient enrollment timelines, as well as enhance retention.

But it is a struggle primarily because a lot of those teams don't own

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the budget. Companies have to communicate internally the value of the patient engagement process to the study teams. And that part is an educational process. At some organizations that comes from the top down. I've seen some smaller companies or companies working in rare diseases do this much more effectively than some of the larger organizations. Larger organizations have so many studies in the pipeline, and so many divisions, that it's sometimes more work to have that communication, and therefore, it often gets neglected.

Steven Taylor

We've also seen a trial fail and recruitment fail - and it would've been important to have patients involved earlier. We've had companies we collaborate with who have to keep pushing the finish line out because they have to beg for patients. Come to find out, their inclusion and exclusion criteria were just too strict.

But companies running clinical trials learn from those experiences as well, which then sells the reason why they should have patient involvement way earlier. Unfortunately, it's not good for our patients when the trial doesn't happen, but it is a learning experience.

Danya Kaye

A lot of companies do learn the hard way. We had a small biotech that asked Inspire to reach out to our Huntington's disease community to see if we could enroll them in this Phase 1 study for healthy volunteers who have the Huntington's gene. I asked them if they had done any market research, because they were under-enrolling. Had they talked

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—STEVE TAYLOR

to this patient population? The response: “Well, no, but they’re healthy volunteers.” The protocol was for volunteers that have the Huntington’s disease gene to travel to a site and spend two weeks there. What, healthy individuals want to use their two weeks’ vacation time to go get blood work when they’re perfectly healthy? It made no sense.

No matter how much outreach you invest in patient recruitment capabilities, your trial risks being unsuccessful if you don’t take the time upfront to really understand what patients are willing to do for a—hopefully—better treatment for their disease. It’s time we demand that our industry focuses on true patient-friendly studies.

Key Learnings

Boost patient confidence by considering every voice—not just the loudest

The voice of every patient and caregiver needs to be heard. The "squeaky wheel" can often be the only voice considered when receiving study feedback, but study teams need to be more cognizant of encouraging participation and input from everyone involved in a trial-- whether in surveys or discussion forums.

Maximize impact of patient and caregiver feedback by planning for the time it takes to implement change

Receiving patient feedback without allowing enough time to take action on the collected data is a missed opportunity to better inform study design. Collecting patient feedback can't be just "checking a box" on a to-do list. It needs to be about making actionable changes to make studies more patient-centric.

Increase the collection of quality data to improve patient-centric study design

Whether the creation of case studies to identify points of burden, a registered patient database to identify eligible patients, or past trial results for better site selection, the more data and insights available, the more informed the study design and protocol can be for future trials.

Key Learnings continued...

Incorporate caregiver perspectives to improve patient input

Caregivers need to be considered as equal participants in clinical trials. Patients may not always have the clearest perspective or be able to tell the most accurate story of their experience. Whether a caregiver is the parent of a pediatric patient or the child of a geriatric patient, their outside perspective is essential to telling to "full story" of the experience that the patient themselves may not be able to tell.

Enhance protocol design by working with Patient Advocacy Groups at the outset

Patient Advocacy Groups are key players when trying to design patient-centric studies--especially in the rare disease space. Involving Advocacy Groups, before trials are even designed, is essential to learning where the potential points of burden may be for specific patient populations.

Visit the WCG Institute to read additional discussions from the Patient Advocacy Forum

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