FDA's recent patient engagement efforts

Pillars of patient advocacy in genomic medicine

The miraculous path to diagnosis for an ultrarare disease

How to treat patients like partners, especially in a pandemic
Welcome Letter

Welcome to the inaugural issue of Patients as Partners.

Building on the success of the Patients as Partners US and EU conferences, this newsletter highlights the many voices that are doing incredible work to vastly improve the patient experience and involvement in medicine development. Enjoy this first issue!

In the newsletter’s inaugural issue, we are pleased to share the following interviews:

**Tina Aswani Omprakash**, patient advocate and blogger, discusses her work publicizing the benefits of clinical trials from a patient perspective.

**Chip Bell**, PhD, customer loyalty guru, talks about the ways pharma companies can engage with their patients throughout the COVID-19 pandemic.

**Fernando Albertorio**, PhD, advocate and entrepreneur, discusses his advocacy in the Hermansky-Pudlak Syndrome and visually impaired communities.

**Christeen Moburg**, Head of Patient Advocacy at Sangamo Pharmaceuticals, highlights the pillars of patient advocacy in the genomic medicine community.

**Neena Nizar**, EdD, Founder and Executive Director of the Jansen’s Foundation, discusses her path to a Jansen’s Metaphyseal Chondrodysplasia diagnosis and starting a rare disease foundation.

We also include coverage of the Patients as Partners 2019 FDA panel, featuring representation from CDER, CBER and CDRH; and a write-up of a Patients as Partners advisory meeting on the challenges COVID-19 has posed and the opportunities for patient empowerment.

Enjoy the inaugural issue.
What’s Inside

4  Tina Aswani Omprakash, patient advocate and blogger, discusses her work publicizing the benefits of clinical trials from a patient perspective.

6  Chip Bell, PhD, customer loyalty consultant, talks about the ways pharma companies can engage with their patients throughout the COVID-19 pandemic.

8  From the Archives: Patients as Partners 2019, FDA patient engagement efforts, featuring representation from CDER, CBER and CDRH.

9  Fernando Albertorio, PhD, advocate and entrepreneur, discusses his advocacy in the Hermansky-Pudlak Syndrome and visually impaired communities.

12  Christeen Moburg, Head of Patient Advocacy at Sangamo Pharmaceuticals, highlights the pillars of patient advocacy in genomic medicine.

14  Neena Nizar, EdD, Founder and Executive Director of the Jansen’s Foundation, discusses her path to a Jansen’s Metaphyseal Chondrodysplasia diagnosis and starting a rare disease foundation.

16  Write-up: Participants in a May 2020 Patients as Partners advisory meeting discuss the challenges COVID-19 has posed for clinical research and the opportunities for patient empowerment.

Recommended Podcasts

Click below to listen to podcast episodes that further the conversation of patient engagement in clinical research.

podcast  Engaging with Communities to Educate and Enroll Diverse Populations

podcast  The Democratization of Patient Data Ownership: Returning Results

podcast  Partnering with Patients on Clinical Development Protocols

podcast  Better Strategies to Inform Patients on Clinical Research & Trial Participation

podcast  Chief Patient Advocate/Officer: Driving Culture Change from the Top Down

podcast  Mapping Out the Patient Decision Journey
Tina Aswani Omprakash is a Crohn’s disease patient and an advocate for the chronically ill. She runs a blog, “Own Your Crohn’s,” with the goal of destigmatizing the conversations around chronic illness. For her Crohn’s disease, Tina participated in a clinical trial and research study.

How did you get into a clinical trial?
I started in September 2015, and I think the drug was approved in October 2016.

The way it started was I was so severely sick, I was at the Mayo Clinic, and my doctors here in New York were already talking about this particular medication, but at a much lower dose. This drug had been approved for plaque psoriasis and psoriatic arthritis, so it was already on the market. It wasn’t like it was a brand-new trial.

Mayo had recommended doubling that dose on me. They were spearheading a trial on it. They said, “We’ll enroll you; we’ll get this all set up. There’s going to be no effort on your part.”

Patients look at how much effort we have to make to be in a clinical trial, whether it’s going to a lot more doctors’ appointments, a lot more maintenance. But I actually appreciated that. I think that for a patient who’s severely ill, yeah, it’s a lot of work. But it means that you’re getting better care and avoiding hospital visits because you’re being taken care of so intensely. The care is on another level. There’s blood draws every week, or there’s iron infusions every week. There’s constant monitoring. After three months, I had an MRI; I had a scope after a certain period of time.

Did you seek out your clinical trial five years ago?
I actually did not seek it out. For me, I go to premier research institutions, where clinical trials have been brought to my attention and are done a lot more frequently at these research hospitals. So in 2010-2011, the same medication was actually brought to my attention, as well as others.

And I said, “No. I can’t be the guinea pig.” I didn’t want to be that. But looking back, had this been presented to me as, “This could change your quality of life, this could potentially work. We have seen X, Y and Z in other patients thus far.” So if there was more transparency around, “Okay, we are seeing this. We can’t promise, but we will take really good care of you. This is a good hope for you to avoid potential further loss of bowels, or surgery.” If someone had presented it like that to me and told me this could really change the future of the way we do things, by having patients participate, I think I would have definitely reconsidered my decision, at the very least.

I don’t think doctors are presenting trials to patients as readily, because especially in the inflammatory bowel disease space now, there are so many more medications that are FDA-approved. There’s at least four or five biologics that are approved; there’s new classes and medications coming in; that it’s almost hard to say to a patient, “I think you should start on XYZ clinical trial,” when there’s five other options on the table. A lot of them are not bringing this to our attention.

What’s a misconception patients have around clinical trials?
I don’t think patients realize how heavily monitored a clinical trial is. I don’t think that’s readily advertised. Yes it’s a pain to go to your doctor that often, but I actually prefer that because then I received better care. Yes, we have a lot of paperwork to fill out and a lot of stuff to take care of on our end and daily symptoms, but I don’t think patients realize how much is also taken care of by the doctors, by the clinical trial coordinators, the hospital system, how I didn’t really see anything from an insurance angle, even though it was billed to my insurance because the medication was approved on some level. I didn’t see any bills; I didn’t see anything that would be concerning to me.

These things are taken very seriously; any small side effect that you have gets reported automatically to the FDA as part of the clinical trial process.

Patients need to hear that they’re in the driver’s seat when it comes to clinical research.
Starting on medication – it’s a lot of biological agents in the Crohn’s disease world – is very scary. You might be seeing the doctor maybe once a month; it’s usually every two to three. When you’re in a clinical trial, it’s so much more controlled of an environment. It’s so much more heavily supervised.

There’s just this fear around trying a medication and being a “guinea pig,” that they’re not looking at how much is actually going into this, and how much time the physicians and coordinators are spending on making sure that the patients are okay and doing well on the medication. I don’t think a lot of patients know that they can drop out of the study, too.
How can we increase knowledge of clinical trials?

For the last year, I’ve been doing a lot of work with clinical trials, promoting them, promoting the awareness of clinical trials in the Crohn’s and ulcerative colitis world, specifically because we have such low enrollment. There are all these medications that are hitting the pipeline, but no one’s enrolling. There are only certain types of populations who are enrolling. We’re not getting diverse populations enrolling. It’s very, very tricky.

There needs to be more social media campaigns about this. There’s a clinical trial awareness day that I did a campaign for in May 2019. I think that needs to turn into a whole week or a social media blitz, whether it’s pharma, or companies that facilitate the process.

I’ve worked with Sanguine Biosciences, Antidote, Parexel; some of these companies that connect you to clinical trials, doing that, as well as patient advocates creating that awareness around clinical trials.

Outside of an awareness week, I think there need to be a lot more patients talking about their experiences.

That’s what I’m doing a lot of, whether it’s on video, through photos, through blog posts: I’m talking about my experiences and what they did for me, how a clinical trial changed my life, and why I was so inclined to partake in a study after that, because I realized how closely monitored and how safe it actually is.

If they see any sort of adverse effects, you could be pulled out or they deal with it right away. It’s so much more closely monitored than some of the agents that are already out there, and medications that are already out there and approved, which I also have had poor reactions to; they have not been taken care of as readily. I talk about this stuff.

That’s what clinical trials are there for: the future.

What can advocacy groups be doing?

I think this is where Patients as Partners really comes in, and I think patient advocacy groups really need to start doing more and more of this.

For instance, the Crohn’s and Colitis Foundation, whom I work with heavily, set up a clinical trials ambassador program. Some of these advocacy groups and nonprofit groups really need to be involved in shining light on what clinical trials can do for patients.

I think this is a multi-pronged approach, but it needs to be done in a way that connects to patients authentically, that doesn’t feel like, “Oh, pharma just wants to be involved.” I don’t know why, and I definitely used to feel this way a few years ago, that pharma is the bad guy. I don’t think so.

There’s a lot of research that needs to be done. There’s a lot of recruitment that needs to be done. There needs to be a greater reframing of what pharma is doing, and how they’re working with patients and patient advocates to bring forward treatments and better quality-of-life for patients. I think that needs to be worked on in the process.

So it takes patient advocates who they trust, and patient advocacy groups who they trust, to bring forward this information, in infographics, “Here are some of the myths around clinical trials,” “Here are some of the truths around clinical trials,” small infographics they can post online. Or stories that can be posted online, things that are visually appealing, but also auditorily appealing for them, where they can feel that there’s hope for them.

That’s what clinical trials are there for: the future. I think there needs to be a lot of reframing, a lot of myth-busting, with clinical trials, and people really need to understand the nuances.

What do you think it would take for patients to participate in clinical trials?

Patients need to hear that they’re in the driver’s seat, when it comes to clinical research.

If a doctor is sitting down with you and saying, “This is a shared decision between you, me and your family for you to enroll in a clinical trial. I’ll let you think about it, but also here’s what I can present you with as what we’ve seen in our institution.”

But you’re really in the driver’s seat. By partaking in something like this, you can change not only your future, but also the future of how we take care of patients.

Maybe having pamphlets in the doctor’s office about the general process of how clinical trials work. Maybe there needs to be some kind of visual in the office and a pamphlet that shows how the clinical trial works, so that the patient can really imagine the future of what it would look like.

There is a real fear around placebos and inclusion/exclusion criteria. Especially in my disease area, the inclusion/exclusion criteria – unless you go to a really fancy institution – you’re not getting into a trial with as severe disease as I had.

Oftentimes, there were trials that I asked about later on that I couldn’t get into because I have an ostomy bag, or I had several fistulas, which are very complex aspects of the disease, and I’ve had several surgeries. So patients like me generally don’t get into clinical trials very easily, unless you go somewhere like Mayo Clinic or Cleveland Clinic, where they can get you in because there are no other options for you.

Inclusion criteria needs to potentially be expanded if they want to include more patients. I understand that’s taking on more risk, but there are patients with more severe classifications of disease that would seriously consider a clinical trial because they don’t have as many options left. 

That’s what clinical trials are there for: the future.
Chip Bell, PhD, is a guru in customer loyalty and service innovation across many industries.

Do you think the pandemic changes customer service in clinical trials?

In terms of customer experience principles, no. I think they remain the same; however, I think there are certain components of those principles now that I will say are sharper, more critical, and more important. Because now, not only do you have a patient who’s coming in anxious about their specific malady, but globally, they are worried about more than that. They’re worried in general.

We start out upset, worried, concerned about COVID-19 and “What’s going to happen to me?” and “What’s going to happen to my family?” We are in a milieu of fear. And so, to me, that even more dramatizes my particular issue that I’m coming to a clinical trial to help benefit. I think it just sharpens those principles in a much greater way.

If I put on the lens of “This is my partner,” how does that frame change my actions as it relates to that patient?

Clinical trials are adjusting protocols, suspending or halting or halting them temporarily, which can be incredibly disruptive to patients. How would you recommend sponsors communicate those changes to patients? To trial staff?

I think it increases the frequency with which you’d need to communicate. It increases the requirement for empathy and compassion. If there was ever a time in which we would not want “clinical detachment,” in terms of the patient and the relationship, it’d certainly be now.

I compare it in some ways, while it might not be a fair comparison, to an Olympic hopeful.

You’ve been training your whole life for an Olympic event. And now the Olympics not only has been postponed, altering your training to your peak, but also is in jeopardy of whether they’ll still be available. I think understanding, empathy, more communication, higher-quality information: all of those now come into play.

Are there other industries that are doing a good job in communicating to their customers that we could take lessons from?

Some that come to mind, that are obviously important in my life, are airlines. I’m not flying, and most of us are not, but I get a note from Delta and American about every week. What they’re focusing on is helping me with ideas about what I can do as it relates to travel.

They are keeping me very detailed up-to-date on changes they’re making to the airlines to make it safe.

That kind of information, at that point which I need to fly, leaves me with much greater confidence. The interesting part, and what makes it more important, is where it’s coming from. Almost all of it is coming from the CEO. So if, at Delta, it’s coming from Ed Bastian, that tells me it must be a pretty big priority for Delta Airlines.

The hospitality industry, Marriott for example, is doing a lot more communication. They’re also putting out more short YouTube videos that give guests a look at what they’re doing. It gives me confidence; it gives me a readiness, at that point at which I’ll be traveling again.

So that constant communication, even though you won’t be doing that activity in the near future, builds trust for when you do eventually fly, for example?

The whole theme is “How do we treat patients like partners?” When I talk to physician groups, I say, “What if this patient was a professional member of your clinic? Another physician on your team?”

Or if you’re a law firm, a partner in your law firm: how are you going to treat this person? What if you treated the patient with that same level of attention, compassion, trust, and focus?

Because we know in the medical field patients are not always viewed from a perspective of equality; they are subjects, so to speak.

The whole orientation of, “Okay, if I put on the lens of “This is my partner,” how does that frame change my actions as it relates to that patient?”
Why do you think the customer has become much more a part of the conversation over the years?

Several reasons. For a long time, organizations, in general, were focused on “If I make a good product, deliver a good service, all other things are going to take care of themselves.” The focus was inward on what they do, what they produce, what they create, without recognizing the power the customer now has that has gone up exponentially, driven in part by social media.

Also, the recognition that the loyalty of a customer is critical; not only in terms of them coming back – that’s obvious – but in bringing all of their friends. I want customers to be my advocate out there, talking me up and saying great things about the service or product that I produce. I don’t just want customer retention; I want customer advocacy, because I know that impacts my bottom line.

If they bring back four other customers, because they say, “You’ve gotta try this,” it becomes an extension of my salesforce, so to speak. Organizations are recognizing the return on investment for investing in a customer’s experience. The relationship has a big, bottom-line impact.

Customers today can also trash your brand. You could be the subject of a YouTube snarky video and damage your reputation overnight. They are much more powerful today. They’re connected like they’ve never been before, and because of that, they don’t just influence the neighbor over the backyard fence anymore. It’s not just “word of mouth anymore;” it’s “word of mouse.”

How do challenges like the COVID-19 outbreak change the way customers and companies, or patients and sponsors, interact?

All of a sudden, all the news is about this. And not only is that subject dominating the news, and what we hear every day, but the words “clinical trials” are a household phrase. We’re all hearing about how long it’s going to take, when they’ll have a vaccine.

The language of that profession is now a much more familiar one to the general public; we’ve all gotten educated. We all know, “We’re in Stage 1,” “Stage 2.” People can give you the stages of a clinical trial, and the timeline of a clinical trial, but I bet you six months ago, they wouldn’t have been able to do that.

The education of the public has brought a new awareness of the pharma industry.

How can pharma companies capitalize on that new general knowledge?

It provides a great opportunity for changing the publicity around clinical trials. It may make recruiting a lot easier, because now we hear these heartwarming stories about some medical student who’s decided to be part of a clinical trial, just because he cares about the outcome that might be produced from that.

The heroism that’s coming out of this, and will continue to come out of this, can’t do anything but make you look different in the eyes of the general public. It may make retention easier, because we all know it’s hard to get patients in a clinical trial, and it’s even harder to keep them in a clinical trial until it’s over.

It is a marketing, publicity opportunity that I think a pharmaceutical company would be smart to capitalize on, and keep that drum going, long after this is over with. Obviously, there’s a self-serving dimension to it, but there’s an altruistic side to it as well.

I tell my friends what it costs to bring a drug to market, and how long it takes and they say, “I had no idea. No wonder that drug costs so much.” So, when you look at a drug and think, “Hope you like it, because it cost $2 billion and 16 years to bring to market.” Before this, they had had no clue of the scale of what’s involved in this. And I think now they will.

I would keep educating the public on “Here’s the altruistic end that we’re trying to provide,” because the pharma industry has gotten a lot of negative impact in terms of costs; a certain sector of the political scheme wants to throw the pharma companies under the bus for making such an enormous amount of money, and “How could my pill cost so much?” But the more education, the more that tends to be looked at in a different way.

The words “clinical trials” are a household phrase. The education of the public has brought a new awareness of the pharma industry.

What would you encourage companies to continue doing after this passes?

I have a friend of mine who follows the workforce trends; he’s predicting after all of this is over, 50% of the workforce will be working from home. Even if the number is off a little bit, that’s pretty dramatic.

I’d be thinking about, if that’s true and we have more of a stay-at-home workforce than ever before, what changes would that make? How does that affect meetings? How does that affect gatherings? How does that affect keeping people informed?

It changes how we look at childcare, how we look at working hours; it changes all of that. Some organizations have always had to deal with that, where they have people working all around the world, different time zones.

I just wrote an article about remote leadership: how do you lead people who are now working at home? It’s not a new thing, because we’ve always had people working on the graveyard shift, so to speak. But I think the amount of it, and the impact it has, will be much greater than it’s ever been.
A Summary from the 2019 Patients as Partners Archive: FDA Patient Engagement Efforts

The panelists were:

**Anindita Saha**, Director, External Expertise and Partnerships (EEP), Center for Devices and Radiological Health (CDRH)

**Megan Moncur**, then Senior Advisor, Science of Patient Input, Center for Biologics Evaluation and Research (CBER), FDA

**Michelle Tarver**, MD, PhD Director, Patient Science & Engagement, Center for Devices and Radiological Health (CDRH), FDA

**Pujita Vaidya**, former Senior Advisor, Patient-Focused Drug Development Program, Office of the Center Director, (CDER), FDA

Patient-Focused Groups

CBER facilitated some patient knowledge through patient-focused drug development meetings, as well as through listening sessions, which are often requested by review staff, using that information to inform the clinical context for drugs and biologic reviews, as well as using it as the basis for some of their research projects.

Switching to discuss CBER’s patient-focused initiatives, Ms Moncur highlighted the “Patient Engagement Workgroup.” It includes representatives from clinical review teams, the policy team, the communications outreach team, the business team, to discuss what is going on across the agency and within the center in terms of patient engagement. In line with CBER’s work in rare disease, they also have a Rare Disease Coordinating Committee, focused on advancing development of therapies for rare diseases and engaging patients in the rare disease area.

Recent Projects - Postmarketing Drug Safety & Mobile App

A recent project of Ms Moncur’s involved leveraging two existing resources to test the feasibility of this – electronic healthcare data that the FDA uses to monitor postmarketing drug safety, and a mobile app platform, developed by the FDA with a PCORI grant – identifying a patient cohort to follow longitudinally.

The goal was to answer the question of whether or not the patient perspective is representative of clinical and demographic diversity and to test the mobile app’s capabilities of recording PROs and patient perspectives.

“If we have that longitudinal cohort, we can start to look at things like how preferences differ,” said Ms Moncur. “So often, we’re capturing preferences for an emerging or hypothetical therapy. What do preferences look like before patients try the therapy, and what do they look like after?”

Ensuring Patient Access to New Medical Devices

CDRH ensures patients have access to safe and effective medical devices, including digital technologies. Since 2016, its mission and vision has focused on how the center can partner with patients to help facilitate innovation with those medical devices, both therapeutic and diagnostic.

CDRH established the Patient Science and Engagement Program to foster integration of the patient perspective in medical device development, evaluation and surveillance. The center created the Patient Engagement Advisory Committee, composed solely of patients, caregivers and advocates.

In 2017, the committee had a meeting about patient engagement in clinical trials, such as how to have patients involved in trial design, the conduct of trials and the communication of trial results. The recommendations that the committee gave led to CDRH’s commitment to clarify and debunk myths of engaging with patients in medical device development.

Patient and Caregiver Connection

Another mechanism developed is the Patient and Caregiver Connection, established in 2018. At the time of medical device review, device companies can facilitate the input from patients in a systematic way that can inform evaluation of a novel device.

CDRH has been working collaboratively, both externally and internally with research partners, to ensure that the patient perspective is encouraged in integration from design to protocol to clinical study to surveillance.

Pujita Vaidya, formerly CDER, spoke about FDA-led, disease-specific meetings as something they’re continuing internally; they had two the previous year to get more information on the opioid crisis, one on opioid use disorder and the other on chronic pain. CDER has also externally led patient-focused drug development meetings as an opportunity to pass the baton and have external stakeholders take charge, follow the FDA model and conduct meetings.

All of the efforts the FDA had made, Ms Vaidya said, were to think about the question: “How can we successfully integrate the patient perspective and input into our overall drug development programs and regulatory decision-making?”

“For all of us,” she continued, “it’s really about how to best foster relationships and how that input can inform regulatory decision-making.”
Advocating and Innovating for Hermansky-Pudlak Syndrome

Fernando Albertorio, PhD, is an advocate for increasing awareness and treatments for managing Hermansky-Pudlak Syndrome (HPS), an extremely rare autosomal recessive disorder, characterized by albinism, visual impairment, and a platelet dysfunction that results in prolonged bleeding. Dr. Albertorio is co-founder of Sunu, Inc, which creates wearable, IoT and mobile technology that empowers independence for people who are blind or visually impaired.

Can you tell us about your background and work?

I’m a serial entrepreneur and I have a science background. I’ve been working in a few different areas in science; my degree is in chemistry. I have a postdoc in physics from Harvard. I’ve been working in business since 2010, creating technology companies in a few different areas.

What is Hermansky-Pudlak Syndrome?

HPS is a genetic metabolic disorder. It’s characterized by albinism, vision impairment, plate dysfunction that also results in prolonged bleeding. As a genetic metabolic disorder, it’s quite complex. There are about 10 different known types of HPS, and symptoms can vary from mild to severe. The severe symptoms can range anywhere from inflammatory bowel disease to pulmonary fibrosis and kidney disease.

HPS was discovered by Doctors Hermansky and Pudlak. It was discovered in Czechoslovakia in 1959 but it’s found across the world. Originally, I’m from Puerto Rico. The island of Puerto Rico has the highest prevalence of HPS and albinism. I was born with the condition of oculocutaneous albinism.

In the severe cases, depending on the type of HPS that you have, you could develop other conditions, like inflammatory bowel syndrome, pulmonary fibrosis. It can develop throughout the patient’s life, sometimes as early as teenage years but commonly later on in life. That can result in a lung transplant.

Can you tell us about how you got involved in your first clinical trial?

My background is in science; I studied chemistry. I went on to work at the National Institutes of Health when I was early in my career, to get more experience at the bench and in research. While I was still getting used to navigating around, I ran into one of the leading researchers in HPS, Dr. William Gahl. He followed me around for a little bit in the hospital, trying to figure out if I was a patient or staff or working at the institute.

I had a very interesting conversation with him, because I didn’t know about HPS at the time. This was back in 1999. He had his laboratory and research group a few floors below where I was working. He shared a lot of information about the condition and invited me to take part in a study. To have the opportunity as a scientist to learn more about the science of my condition, it was great to learn from Dr. Gahl and his staff.

What was it like to learn about HPS from your viewpoint as a scientist?

It’s very interesting. So, at that time, I didn’t know anything about patient advocacy. I had recently graduated with my degree in chemistry, and wanted to focus on getting into grad school and deciding on if I should go into medicine and become a doctor. I wasn’t sure about my career.

Learning about HPS through Dr. Gahl and his team was a life-changing experience on many levels, both personally and professionally. Getting to experience the protocol, being a patient in a clinical trial, meeting families from Puerto Rico and other people with albinism who were there to get tested or go through the trial as well – it was a very eye-opening experience for me.

I started learning about advocacy primarily by talking to patients or talking with family members, discussing the science behind it, trying to explain it. It was my first entry into advocacy.

Being a scientist, my curiosity about how the disease works gave me some understanding about the molecular level and the genetic level. It gave me a little bit of comfort knowing that there was science being done and that we were diving into how HPS works genetically, and how ultimately we could get to a cure. Being able to explain that in a way that other people could understand was a great experience.

Being a scientist, my curiosity about how the disease works gave me some understanding about the molecular level and the genetic level.

What is the HPS treatment landscape?

The symptoms can vary from mild to severe; I have a mild form of it. But for those people who have more severe forms of HPS, it involves regular checkups with...
pulmonary physicians to monitor pulmonary function. You may be on certain medications to delay the fibrosis of the lungs. Ultimately, if the condition progresses further, you have to prepare for a lung transplant.

It requires continuous medical care and attention, regular visits to the doctor and living through the other conditions; it can be quite debilitating.

Especially during this time of COVID-19, it’s really an eye-opener to think about how we live. Everyone is concerned about if they get sick. For people with HPS, and those with the form that affects respiratory function, it can be especially nerve-wracking.

Imagine going to the hospital, figuring out that you’ve got COVID-19. On top of that, you have HPS. You have to explain that to the medical staff there and face the potential outcomes if you were to go into the ICU.

**Can you tell us a little about the HPS Network?**

The network’s founder is Donna Appell; her daughter has HPS and one of the first patients in that trial. They were really instrumental in getting the researchers at the NIH to pay attention to this and develop a trial with Dr Gahl’s team and other teams.

Donna and her family formed the HPS Network as a way to provide education for people with albinism and their families, and raise awareness of Hermansky-Pudlak Syndrome.

Not all people with albinism have the gene for HPS, but it’s important that, if you are a person with albinism, to get tested and to learn about the condition that could affect your life later on.

They were very instrumental in developing educational experiences for people, going into Puerto Rico, creating day seminars and conferences. The network is also an advocate for people with albinism and HPS. They promote the scientific development and research for the condition, for the purpose of a cure.

Because that’s what we’re looking for, finding a cure, especially for those who have the more severe forms of HPS – improved treatments for pulmonary fibrosis and for HPS. I’m very grateful for their work.

**You’re also an advocate for people who are visually impaired. Can you tell us about that?**

I tend to advocate more for people with sight impairments, either blind or low-vision. I became involved in the HPS Network by meeting Carmen Carnacho, who is on the HPS Network board of directors, in the Boston area and their New England, primarily through social activities but also by trying to help out anywhere I can.

Today, I combine my science and entrepreneurial experience to my advocacy work. My advocacy work involves folks with low vision and the use of technology and product development towards empowering the lives of people with disabilities.

**What kinds of tech are in the space?**

As part of my advocacy work – this happened as a progression throughout the years as a scientist and an entrepreneur – I co-developed a company called Sunu, that creates mobile, wearable and IoT technologies that enhance the lives of people with vision loss, blindness or low vision. The technology is a wearable device that uses sonar with haptic vibration feedback to improve the individual’s awareness of anything in the environment. It alerts the user to anything that could be an obstacle and helps to reduce accidents. The Sunu Band is helping people practice social distancing.

As part of my journey as an entrepreneur, I’ve been developing as an advocate; I feel that the two go very well together. As a technology creator and as an entrepreneur, we have to be active in our communities and with the people we want to serve, and understand not just how to add value through a product but how to enhance that value through conversations and advocacy.

**How are you engaging people in your advocacy?**

I’m hosting a video series where I provide information and content through conversations with leaders, experts and advocates in the community, to help us better live through COVID-19. How we engage our community during this time, like going out to the supermarket and using technology to maintain social distancing.

We’re also uncovering a lot to improve – access to materials that are normally in print, and make them accessible for low-vision and in Braille for those who are blind, and in audio form. Things like signage in the supermarket, labels on food. As a community we’ve been asking for this to be fixed for many years. Right now, the pandemic has created an urgency to apply design, apply a little bit of universal design, to solve some of these gaps.

**How do you think the pandemic pushes forward conversations for people who manage rare diseases or might be visually impaired?**

This is a conversation that needs to continue to happen, and it needs to be in the forefront. For people who are leading and managing, it’s an opportunity for them to innovate, to get creative, to form partnerships with technology companies. There are quite a few startups that are nimble, that can reengineer, that can use design to bridge some of the gaps that we’re talking about.

We should also provide information and advice about how you advocate for yourself, if you were in the hospital or a similar situation, as a patient with a rare condition or a vision impairment. Imagine being in a hospital and not being able to breathe well, and on top of that you’re visually impaired, and you’re given forms to fill out and sign. Those materials are not accessible.

COVID-19 has opened up an opportunity to have these conversations with the various stakeholders, like medical teams and healthcare, about how to improve that experience for people with disabilities.
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Genomic Medicine POV

The Pillars and Principles of Patient Advocacy

Christeen Moburg serves as the Head of Patient Advocacy at Sangamo Pharmaceuticals, a genomic medicine company. She is responsible for developing and implementing strategic patient advocacy initiatives to advance Sangamo’s patient engagement, business and reputational goals. In this interview, Ms Moburg discusses data return, informed consent and patient engagement in genomic medicine.

What are you focusing on now at Sangamo?

We have strategic pillars for patient advocacy at Sangamo, which are built around delivering the patient and caregiver voice in everything we do, establishing patients as valued partners, and driving education and awareness. Some of this work is internally facing, and some of it is externally facing.

One of the key things I’ve been working on since I joined Sangamo, and that we continually improve on, is how we integrate, and continue to expand, the patient perspective in our development process.

Other areas I’m working on currently include how we return patient data to patients that participate in clinical trials and the consent process. With genomic medicine being such a different type of treatment approach that can have long-lasting effects on a patient compared to traditional small molecule pharmaceutical products, how do we ensure our consent is meeting that higher level?

This ladders up to our corporate values; patient centricity is a primary corporate value. The internal part is integrating patient feedback and experience into the development process. The external part is building relationships with patient groups so that we can bring the patient voice to our internal teams and make sure that the patient perspective guides our research and development decisions. And there is the data and consent portion.

Patient engagement is not just a “nice to have.” It’s a “must-have.”

What are the specific considerations for working in patient advocacy delivering cell and gene therapy?

Being that these approaches are in their infancy, we are looking at the ethical considerations. For Sangamo, that includes three main principles: transparency, informed consent and long-term follow-up.

With regards to transparency, we need to figure out how to return data to patients and that the way their data will be used is transparent and consented. For informed consent, we need to make sure clinical trial participants fully understand consent given the long-lasting, potentially lifelong effects genomic medicine may have on a patients’ DNA, and also that they understand the commitment the company makes to them. Lastly, for long-term follow-up, we need to understand the long-lasting effect that genomic medicine product candidates may have on patients in order to responsibly develop these medicines, and therefore need the ability to follow them in the long-term.

When you’re going into a genomic medicine trial, it’s not like in two years, the trial is over and you’re done. We have requirements from the FDA for 10- or 15-year follow-ups. Those are all things that are new and changing rapidly in the genomic medicine space. As we do our work, we have to make sure we’re engaging with the patient community across all of those aspects.

I work with the patient communities to help educate them on these new therapeutic approaches. It is important that they understand genomic medicine platforms, gene therapy, gene editing, cell therapy and what these new medicines might mean for them. They understand their disease better than anyone else; when I first started in pharma, you were educating people on their disease. That is not the case in rare diseases. You’re educating them on their options, but patients know their own disease best.

In the development process, what are the early considerations of concretely and practically incorporating the patient voice?

Our company mission is translating ground-breaking science into genomic medicines that transform patients’ lives. To do that, we have to ask patients for their help in the process. As we go through our development process, our scientists develop potential targets for treating a certain disease based on our understanding of the genes involved. Sangamo is the first genomic medicine company, meaning we can do gene therapy, cell therapy, genome editing and genome regulation. There are many potential gene targets that could be a good fit for our technology capabilities, but that doesn’t mean that the patients impacted by those diseases need or want a genomic medicine. That’s why we’re asking for patients’ help in the earliest stages of our research programs and that we’re gathering insights from them that are going to inform our R&D process.

In genomic medicine, we’re talking about a very different bar, because these medicines could potentially be curative or really a game-changer compared to either existing treatments or no treatments. But when we engage with patients, we ask them questions that are
going to inform our decision on whether or not to move forward. We need to understand their current standard of care. We need to understand their disease journey. We need to understand their unmet needs. And we need to understand what the quality-of-life or meaningful endpoints are, from their perspective.

Those are all the things we’re thinking about when we say, “Patient Voice.” It sounds good, but are you really doing something actionable and concrete? That’s the thing that I feel is so important. When we’re planning on the “How” and “Where,” it needs to be at a point in time where it’s going to impact the decision. It can’t be an afterthought to check with patients when the decision has actually already been made.

How does working with patients from rare disease or small patient populations change consent?

It’s very different, for multiple reasons. The number one being that, with genomic medicine, it doesn’t just wash out. You can’t just stop taking it. If you’ve participated in a clinical trial for genomic medicine, it’s in you, potentially for life. So, the consent process, the approach, the decision, the trial design, the long-term follow-up—it’s a much bigger commitment from the company, but it’s also a way bigger commitment from the patient.

We, as leaders in the space, need to be thinking about it differently. We need to make sure the patients truly understand what they are saying yes to. We have to be thoughtful and mindful, and make sure that we are clearly articulating and conveying, to set clear expectations.

The importance of partnering with the rare disease community is elevated in genomic medicines to ensure we get it right.

We say, “Patient Voice.” It sounds good, but are you really doing something actionable and concrete?

How does it change returning data?

The question of returning data from clinical trials to individual patients is not necessarily specific to the genomic medicine space.

Here are two different examples. One pertains to an oncology patient, where in that case you’re potentially going from trial to trial to trial. You’re going from trial to trial, having very repetitive, invasive testing, because the tests don’t come back to your medical chart. Another example is a pediatric patient who had to have an MRI as part of participation in a trial. And then, for some reason, the child needed an MRI outside of the study, and had to have a second MRI, which in a pediatric patient is done with anesthesia. Because the family didn’t have access to the MRI that was part of the clinical study, they had to do it all over again.

What we’re talking about is how to ensure, at a point in time when it will not unblind or jeopardize the study itself, that the information will go back to the patient’s record. It’s somewhat touchy. Because it’s my understanding that a patient’s medical records are their medical records; they are the owner. That doesn’t matter which country you live in or why those data were collected. And in the examples above, it is not in the best interest of the patient to keep that information from them. My feeling is that we should be doing it already. We just need to figure out that process, and also the timing. Oftentimes, when you hear people talk about patient data in the context of clinical studies, they’re talking about the study data, specifically, of the patient. I’m really talking more about the testing and other specific information that the patient should have access to.

The big difference with genomic medicine is that if they go into a genomic medicine trial, whether it’s gene therapy or gene editing, they may not be able to participate in another trial. Because under the current viral vector delivery systems, you now have potentially an antibody response to the delivery vector. Again, higher bar in terms of consent, higher bar in terms of transparency, and also getting that data back to the patient’s record, that if they’ve had an AAV6, AAV5 or AAV2, that it’s part of their medical record because that could influence their treatment in the future.

Can you speak more to the higher visibility of patient engagement?

Some of that was definitely impacted by the patient groups. The patient voice is powerful–having organized patient groups has really led a positive change. I can remember years ago, when there was an advisory committee meeting for the FDA—they were going to tell the FDA whether they think a drug should be approved or not. Having a patient speak at those meetings was always impactful. But how does a patient know to show up at something like that? You can’t drive them to it; you can’t advertise it. If you’ve engaged with them throughout the process, they become their own advocates and yours if it’s a good product and a good process.

In addition, the higher visibility from the regulatory agencies, FDA and EMA, whom we commend for all of their efforts to bring patients into the conversation has really increased the importance of patient engagement throughout the development process. It’s shown people it’s not just a “nice to have.” It’s a “must-have.”

With all of the changes, like the 21st Century Cures Act, the elevation of the patient voice has been a process. I think about the Clinical Trial Transformation Initiative. That’s from 2007. We’re in 2020. It hasn’t been an overnight change; it’s been a very long process, but it’s been long overdue. During my career, I have witnessed these changes and the ways they enrich patients’ lives because they can be involved in potential treatments and enriches pharma for having their critical input.

One of the countries that we recently had a regulatory submission in not only asked if we had engaged with a patient group; they asked which patient groups and asked “What did you do with the feedback?” That’s great, because it’s not just a “Check the box.” It’s not just a “Nice to have.”
Creating a Foundation and Path Forward for an Ultra-Rare Disease

Neena Nizar, EdD, is the Executive Director and Founder of the Jansen’s Foundation, which was created to speed up and fund the process of finding a cure and treatment for Jansen type metaphyseal chondrodysplasia, an extremely rare progressive skeletal disorder.

Can you tell us about yourself and your work?

My name is Neena Nizar, and I have Jansen’s Metaphyseal Chondrodysplasia. I’m the Executive Director and Founder of the Jansen’s Foundation. I’m also a mom to two boys with Jansen’s disease.

What is Jansen’s Metaphyseal Chondrodysplasia?

It’s an ultra-rare skeletal disorder, caused by an overactive PTH receptor. The receptor is damaged, and doesn’t regulate bone turnover very well.

All the growing ends of our bones are not able to take calcium and make new bone. We turn over bone so much faster than the average person would. The bones are very soft at the ends. All of our long bones are affected. The excess calcium remains in the body and has to go to the kidneys to get filtered out, and can lead to kidney damage if not treated early on.

More than that, Jansen’s also causes a lot of pain because the bones are forever bending. And because of that, weight distribution is messed up. You have weight on your body at different angles; that causes pain. Walking is difficult; day-to-day activities are impacted.

There’s an enormous amount of surgical intervention throughout childhood, so you’re spending a lot of time either in surgery or recovering from surgery or regaining skills to learn to walk.

Right now, there are less than 11 people that we have identified with this disease. Worldwide, since the disease was discovered, there have been only 30 documented cases.

What are the challenges associated with creating therapeutics for a small patient population?

We don’t have that many cases. The way we develop drugs for rare diseases is with a rich, natural history study. But when you have limited numbers, even coming across a treatment or moving a treatment forward is such a huge task, and sometimes the chances are negligible.

That’s why we set up the foundation in 2017, to spur interest in this disease area and find a treatment, not just for my boys but all of these children affected worldwide.

What was your diagnosis journey?

My journey has been pretty incredible. I was never diagnosed with Jansen’s because I don’t present symptoms in the typical Jansen’s manner. It baffled doctors; I was misdiagnosed as a child as having polio, and then rickets and several other skeletal disorders. I was born in the 1980s, in Dubai, at a time when there was no Google and you relied on the doctor to tell you what you had. What he said stuck. We never knew any differently. I went through several surgeries growing up, and several interventions to help my bones.

When I got married later on, I was told I would never have any children. My husband and I were in the process of thinking about adoption when I became pregnant with my first son.

We had no problems with the pregnancy; my son Arshaan was born 9 lbs at birth. There was no inkling of any kind of disease or that there was something wrong. He hit all milestones. We were joyful. We were new parents. We did everything we could to make sure he was healthy and safe. He did really well.

I was pregnant with my second son, Jahan. Three or four months into the pregnancy, my first son started regressing. It was almost overnight. We were upset of course, but having had my experience growing up, I knew that even if it was a skeletal disorder, we could still give him a very good life.

In our minds, we thought that if it was a skeletal disease, we could manage it the way my parents managed it. At that point, doctors began noticing things like his kidneys, and that his potassium and calcium were really high. They started noticing things that confused us because I had no history of them.

I was going back, looking at my records, trying to make sense of it. We wondered, “Is this a different condition than what I have?”

That was the scary part, just not knowing.

She took one look at my son’s X-rays and she said, “I think I know what you have.” Of course, it was the moment that changed our lives.
What did you do?
At that point, I quit my job. My whole focus was on figuring out what this was. We finally stumbled into the office of a pediatric geneticist in South India, Dr. Sheela Nampoothiri. We walked into her office; she took one look at my son’s X-rays and she said, “I think I know what you have.” Of course, it was the moment that changed our lives.

How did Dr Nampoothiri diagnose you?
It’s quite magical and miraculous. She had studied rare skeletal disorders in Germany. While teaching the class, her professor had shown one slide and said, “We’re going to skip this slide because you’re never going to come across a Jansen’s patient in your life.” When she saw the X-rays, she remembered the slide. She called up the professor and said, “I don’t have just one patient with this disease. I have three.” Our blood was sent from a lab in India to the US. Within a few days, we had the diagnosis of Jansen’s disease confirmed.

What happened next?
We knew that the boys needed care and treatment that wouldn’t be possible in Dubai. We knew that we needed to come to the US. My husband is American; he had lived in Nebraska. So when we moved stateside, we moved to Nebraska in 2015. Upon researching the disease, we found very few articles about it. Most articles were written by Dr Harald Jueppner and Dr Thomas Gardella.

Our first hunt was to find Dr Jueppner and his team to talk more about the disease. Very soon after, we realized that they had all the research, a mouse model and some theories about the disease that they had shelved because there were no patients and no one to drive it. Here we were: myself and two boys.

We had such rich data because I’ve lived the disease. I have my notes. Everything I can share. It was a collaborative endeavour with Dr Jueppner’s team. They had researched it for 20 years and never met a patient. Then they met us, and now they had so much more impetus to take some of that research off the shelf, dust it off and start on treatments. Something to lessen the surgical interventions and the pain.

What is the treatment landscape for Jansen’s?
Right now, everything is so much more hopeful than when we first landed in 2015. In 2017, we set up the foundation, and by the end of 2017, we got the R01 grant from the NIH to pursue treatment options and research for preclinical data for Jansen’s. What Dr Jueppner’s team is doing is really putting together a ligand, an inverse agonist, really, to turn off the overactive PTH receptor. They had very good data in their preclinical models and their mouse models that the NIH felt that we had a strong case to move forward to a clinical trial.

At the end of 2018, we received the TRND grant from the NIH, which is the Therapeutics for Rare and Neglected Diseases, to help us with toxicology studies and the production of the material to go into a clinical trial. We’re really on course for the first-ever trial for Jansen’s.

What could be done to better support patients with ultra-rare diseases?
When I first started, one of the things that I was told was that nobody would care about eight patients in the world – we were at eight patients when I first journeyed into the rare disease space to find people who’d be interested in helping us. That was the first thing I was told.

The first thing we need to do is change our mentality. We have to change the language we use around “rare.” It doesn’t need to be so defeatist. It doesn’t need to be so declarative in a negative sense or crushing of all hope.

We’re in a time when gene therapy is taking off. We have so much more hope finding treatments for rare conditions. Precision medicine is at the forefront in today’s rare disease space. I don’t think anyone needs to feel, “Well, we don’t have enough people,” or “We don’t have enough history.”

In the larger scheme of things, there will be someone out there researching or who has an interest in looking at what you have. What’s important to look at is that there are so many startups; there are so many people entering the space, wanting to do good and help. Build your relationships and collaborate wherever you can, with the purpose of moving treatments forward.

I think a lot of people get stuck in the roots of it all, as in, “How do we set up a foundation? Do we need to do this and that?” That’s very time-consuming. When you don’t have time on your side, you have to plot the fastest path forward.

We’re in a time when gene therapy is taking off. We have so much more hope finding treatments for rare conditions.

What would you tell other people with rare diseases?
I don’t think we should feel that we are powerless. People with rare diseases grapple with a lot, in terms of treatment and understanding the disease, living it and then being able to circumvent all those emotions to plot a path forward.

Being able to see clearly, even though you’re so weighed down by so much emotion, is going to be the way forward. The space has been fabulous; it’s very collaborative in nature. NORD and Global Genes do such a great job of supporting.

Also, lean on advocates who have gone before us in rare disease, people who have run foundations; always lean on the experienced voices in the field. Don’t get pulled down into the weeds of it all; make sure your path is very clear. ◆
In May, participants of a Patients as Partners advisory meeting discussed the challenges COVID-19 has created, as well as the opportunities for patient empowerment.

The advisory meeting included representation from biopharma and patient advocacy.

The COVID-19 pandemic has upended life in every facet but has had a particularly halting effect on clinical trials and patients.

“What I’m hearing from folks at cancer centers is that everything is coming to a grinding halt, at least in New York,” said Ellen Miller Sonet, Chief Strategy and Alliance Officer, CancerCare. She noticed that across the board, there had been a significant decline in trials being run.

The pandemic by-and-large shuttered clinical research operations as lockdowns set in. Now, as activities begin to open up, operations have to judge restarting and reopening on a trial-by-trial, state-by-state and country-by-country basis, taking into account various levels of restrictions, regulatory guidelines and the latest COVID-19 activity. It’s a balancing act.

In addition to facing the challenges of running or adapting clinical trials during a pandemic, the industry has a once-in-a-lifetime opportunity. Clinical trials have never been more a part of the general lexicon; there is the opportunity to push that awareness further. It’s just the spotlight has been turned to the industry during a time of intense change.

“For years, we’ve been struggling with raising awareness of the value of clinical trials as a healthcare option,” said Jean Stimola-Sposaro, former Associate Director, Patient Network Management, Sanofi. “Now, more than ever, we’ve raised awareness that we lacked before.”

“It’s in the news. People are using those two words more often than I’ve heard them. It’s an opportunity to support the message, value and importance of clinical trials,” said Pankaj Patel, Director, Clinical Trial Patient Advocacy, Bristol-Myers Squibb.

Now more than ever, patients have to be at the forefront.

Grinding to a halt and pivoting to the future

For the most part, agnostic of indication, current trials have maintained momentum but most new trial initiatives have halted for a number of reasons.

First, as Mr Patel pointed out, was site burden. “It’s difficult for sites to engage with patients in existing trials, let alone have to deal with new patients coming in.”

Second, potential or current participants in clinical trials were finding it difficult to travel to sites for financial, health or safety reasons.

And third, sites and sponsors were having to juggle country regulations, state guidances and an ever-evolving COVID-19 spread.

However, some alternative forward motion was happening in clinical research. Karlin Schroeder, Senior Director, Community Engagement, Parkinson’s Foundation, said that some academic centers were pivoting to virtual visits where possible. “I’ve had a fair amount of industry partners come to us to talk about pivoting their trial to virtual,” she said, “and doing protocol amendments so that they can do the study through virtual visits.”

In other cases around the globe, where the virus hit early, trials are beginning to open back up. As Mr Patel reported from the BMS perspective, that is a trial-by-trial and case-by-case decision.

Tara Hiley, PhD, Director, Clinician, Early Clinical Development, Pfizer, added, “We had a complete ceasing of recruitment for a short period of time, except for a few key protocols, primarily oncology, but now, in the last week or two, we’re opening up again and working with sites, enrolling, working with participants who are uncomfortable going to sites, working remotely as possible.”

During a recent call with the Clinical Trials Transformation Initiative (CTTI) on quality by design for COVID trials, Ms Schroeder spoke about the patient engagement on COVID trials, thinking about the disproportionate impact on certain communities, and the voice of the patient. The purpose was to ensure that the trials are well-designed on the patient experience end, while knowing that time is of the essence.

Schedules are being affected, but where flexibility is possible, according to Marilyn Metcalf, PhD, Senior Director, Patient-Focused Development, Global Medical, GSK, it’s being taken, such as telehealth, taking services to patients or offering options to do more at-home administration of medicines. “Patients are appreciating that there is some flexibility where possible, and hoping that those kinds of options can continue moving forward,” she said.
New opportunities pushed to the forefront

There have been issues discussed for years in clinical research that are now being put into sharp relief. Informed consent documents, for one, as Ms Schroeder pointed out. A forty-page consent document doesn’t pair well with a patient in respiratory distress who might be separated from family members.

The COVID-19 pandemic does present opportunities for engagement and education. The general public has been talking about the process of creating vaccines, and as Ms Sposaro-Stimoli pointed out, this is a moment to educate the public on the guardrails that have been put in place to ensure vaccine efficacy and patient safety.

“This could be a wonderful teaching opportunity to explain what clinical trials are and why, even though there’s such a great need, we must make sure that everyone is safe and that the medicine is effective, and use that to explain the importance of clinical trials,” said Beth Zaharoff, former Director, Patient-Focused Clinical Trial Engagement, GSK.

For years, we’ve struggled with raising awareness of the value of clinical trials as a healthcare option.

Now, more than ever, we’ve raised awareness that we lacked before.

It’s also a chance to accelerate virtual trials, telemedicine and virtual visits, to help patients engage with their healthcare. Making sure that the lines of communication have been clear throughout the crisis has been critical.

That communication can continue, beyond the crisis, to great effect. “There’s so much anxiety because of COVID-19. Whatever we can do to minimize that anxiety, the better,” said Ms Stimola-Sposaro.

Ms Stimola-Sposaro brought up a recent example. Before the pandemic, Sanofi had planned a large, face-to-face oncology patient advisory board to elicit feedback on an early breast cancer trial.

When COVID-19 hit, they were surprised to learn that, even with everything else going on, patients were still so eager to give feedback. One reason was, “It’s nice to know we haven’t been forgotten.” Another was, “Thank you for taking the time to consider us and remember us.”

Bring power back to patients

For a long time, the industry has talked about patient empowerment, and how patients are becoming more empowered from a knowledge perspective.

Mr Patel used the example of how learning more about the spread of the novel coronavirus empowers knowledgable decision-making. In the same way, knowledge allows patients to understand their options. Empowering patients makes them “more party to the decisions that they make,” said Mr Patel.

“How do we make patients part of that shared decision-making about their own healthcare? That is by empowering them,” said Ms Stimola-Sposaro.

“It all ties together really well, in terms of patient engagement and designing research,” said Ms Schroeder, combating misinformation about research and an opportunity for education. She pointed out that if a patient is a part of that clinical trial design process, it demystifies much of the process and takes away some of the fear of participation.

“These are issues we’ve talked about for years, but they’re being forced now. How do we leverage that to make these changes, and make them permanent, as we move forward?” she asked.

“Now more than ever, patients have to be at the forefront,” said Ms Stimola-Sposaro. ✶

Since the May advisory meeting, Ms Stimola-Sposaro has become Associate Director, Global Clinical Business Capability, Lead, Clinical Trial Industry Collaborations at BMS and Ms Zaharoff has become Senior Director of Patient Engagement at Alkermes.
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