Scout

UNDERSTANDING RARE DISEASE:

Challenges & Opportunities in Today's Clinical Research

An interview with KimberLee Heidmann,
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From patient retention to regulatory and ethical complexities, clinical research takes on a unique and challenging form in the realm of rare disease. Join us as we explore the profound impact of patient experience and the pivotal role of patient advocacy groups in achieving clinical breakthroughs with KimberLee Heidmann, Executive Vice President of Patient Experience and Customer Success at Scout.

What defines a rare disease? What makes clinical research in this area unique compared to more common diseases?

In the US, a disease is considered rare if it affects fewer than 200,000 Americans. Other countries will have slightly varying parameters for what's considered rare. There are somewhere between 7,000 and 10,000 identified rare diseases.

From a clinical research perspective, the most important factor is finding the patients. One of the biggest issues for rare disease families is getting a diagnosis. A lot of times people will have symptoms, or parents and caregivers will see symptoms in pediatric rare diseases, but their general doctor may not understand. The doctor may see symptoms that point to a specific rare disease that they've heard of, but a general practitioner or pediatrician might say it's out of their realm, or that it's some other disease or disorder or viral infection. Patients need an appropriate diagnosis, and then they need to be treated or have access to a physician that actually knows what research is available.

Another unique element is that there actually isn't much research out there. Not many companies are researching treatment for rare diseases, and somewhere between 90 to 95% of rare diseases have no available treatment. If there are at least 7,000 known rare diseases, and 90-95% have no treatment at all and are mostly treated off-label, just doing the math on that tells you how much research needs to be done.

How does the rarity of a disease impact the recruitment and enrollment of participants for clinical trials? What are the specific challenges?

First, you need a sponsor willing to do the research to find treatment. In some of these rare disease cases, there is no curative therapy. There are some rare diseases where the disease progression and the etiology of the disease is not as well known, so a lot of the research that we're doing is on disease progression rates. The drugs or the treatment that may be already available are designed to slow progression. Patients aren't going to take this drug and be cured of their disease.

For many families pursuing care where there isn't a cure, they're looking to extend the life and the quality of life for their loved one. Or in some cases, adult participation in clinical research may help extend or increase the quality of their own life, but in a lot of ways, participating in that research is altruistic because it's planning for the future. It's thinking about the impact of that research on future patients and for our loved ones and descendants down the line. So, you've got patients and families that enroll in clinical research because it gives them hope for the future. If there is no other treatment or curative therapy, research may provide an extension of the life that they have with their loved one and the quality of that life.

But specifically and logistically, it's very difficult. Once rare disease patients receive a correct diagnosis, and those patients are qualified for an active study, then they need to locate and travel to where that research is being conducted. A hot topic in research right now

is cross-border enrollment in global clinical trials. With the emphasis on increasing diversity in clinical research, we're trying to find patients all over the world of different socioeconomic representation, or genders and ethnicities, or patients who live in cities versus rural areas, and so on. These factors impact their participation in the trial as well as outcomes. Once you've found those patients and identified where they can be treated, how can you get them to the site for treatment? It's a two-fold challenge.

Speaking of cross-border enrollment, how does the regulatory landscape surrounding rare disease clinical research impact trial logistics?

The cross-border enrollment piece has really impacted regulatory complexity. Let's say there's a trial in a disease that specifically affects people in a certain part of the world. While patients may be affected by the disease in one region, there may be no treatment centers in their home country. How do you get patients involved when they're not actually going to be engaged in the study until they reach the site?

The discussion around how to get consent from the patients, make sure that they're receiving treatment at the facility that can be covered under the clinical research piece of the study, and that they're getting standard of care through their physician at home presents a very unique set of challenges.

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Another major component we're dealing with is data privacy, which is a general global concern, but especially so when it comes to clinical research. Participant privacy is so important—not just for their personally identifiable information, but their protected health information as well. We must design the consents correctly for this unique model, put the right language in the form in a digestible way, and get approval from the IRBs and ECs.

There are a lot of complexities around this that you wouldn't see in a trial where the patient is being consented at a site local to their home, perhaps even in the same hospital with their primary care physician. If I'm moving a patient from Egypt to Turkey to participate in a clinical trial, how am I presenting that data compliance in the ICF so that it can be approved? Or, how are we approaching that while the patient is consenting to share their data with Scout, they're not necessarily consenting to participate in the trial? There are significant regulatory considerations that must be made.

In addition to data privacy and consent considerations, how can researchers ensure the ethical conduct of clinical trials for rare diseases?

Data privacy and informed consent are step one. We have to present the information in a way that is understandable to the patient. They have to know all of the potential benefits and risks. They also need clarity around reimbursement for out-of-pocket expenses, stipends, or time and wage loss compensation. Those types of things have to be very clearly outlined.

Step two, when you talk about ethical conduct with rare disease, there is often a more significant burden on the patient and the family to complete study visits. For example, let's say you have a pediatric patient with DIPG (diffuse intrinsic pontine glioma), a highly aggressive and hard-to-treat brain tumor. Very specific hospitals are treating and researching this, so the patient is potentially going to be traveling long distances for treatment. DIPG patients are severely compromised in terms of needing special equipment just to be able to sustain the travel to the visit. We have to think in terms of an ethical perspective; we have to think in terms of patient need. We must look at the burden on the patient and their care circle to establish what we can do to offset that burden.

In clinical research, the potential for patient services like travel support and reimbursement to be perceived as coercion is serious. In the rare disease space, this should be a very different conversation from a regulatory perspective, because the burden on these patients to actually complete their visits is different from the burden of many other therapeutic areas. In most cases these patients will require more extensive travel support (or Patient Navigator support where they're receiving white-glove, customized service) or more offset of financial burden just by the nature of their disease process and the location of the clinical research facility.

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What role do patient advocacy groups and foundations play in advancing rare disease clinical research? How can researchers effectively collaborate with them?

In my opinion, the most important role that they play is giving a platform for the patient voice and keeping families from feeling isolated. Rare disease families can feel like they've been abandoned by the healthcare system. They may have been telling their providers about their child's or their own symptoms and were told that it wasn't a significant concern—or that it was some other illness, and it's not. When they finally receive an accurate diagnosis, they need support from others who have had a similar experience. Advocacy groups provide a space to be with other people who have had like experiences.

Once you get those people together and you give them a megaphone via an organized advocacy group, they can start to push that voice out to those who will listen. Advocacy groups have made a huge impact on sponsors: listening to what patients need and putting the emphasis on research for these diseases and underserved populations. So firstly, they give the patients and their families a voice and secondly, they push research forward. They're the squeaky wheel saying, “We're finding new information about the disease, we want to know more about how we can help our families, and we want to know more about what our treatment can look like.”

They also provide a wealth of information. For Scout, the advocacy groups are informing our services by telling us what patients and families need, which is going to positively impact our patients and therefore our sponsors because we're going to be able to retain those patients in a trial. We need to do everything we can to make their participation efficient and support them in that. Then we get the data, we get the outcomes, and we positively impact therapy in the future. Sponsors are able to bring the new drugs and new therapy to market, impacting future generations. It all works together, and it starts with the advocacy groups and the megaphone they give to the patient voice.

What effect does the patient experience have on trial success? How can clinical trials be designed to optimize the patient experience?

We need to change our perspective on how we look at the patient and the family or care circle experience in clinical research. In the past, people have had a bad taste in their mouth about research because of the use of the word “subject” or the traditional perspective that as a participant you're a “lab rat.” The whole research industry has changed. We're starting to view clinical

research as healthcare in light of underserved populations that are either uninsured, or underinsured, or don't have access to medical care.

Beyond changing that perception, we also must start looking at the patient and their family and what their experience looks like from a more humanized perspective. Clinical research participants are human beings; they are people of different ages, races, genders, and experiences. When we look at the patient as a whole person, we understand that their experience inside the trial will impact outcomes.

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As employers and business owners, we treat our employees with a great deal of respect: we give them the desks, tools, and technology that they need. We need to do the same for these families and patients who are dedicating hours of their lives to working towards trial success. If they are supported in overcoming the barriers to their participation, that will optimize their experience–which ultimately affects the impact and the outcome of the trial.

In your experience, what are some breakthroughs that have been achieved in the field of rare disease clinical research?

We started Scout in 2016. Even though at that point I had been in the life sciences space almost 20 years, I don't think I understood the degree of influence to which the patient voice and interaction with participants and advocacy groups could potentially have on the clinical research industry. Since then, I've personally had the honor to watch the industry evolve and witness the conversation that thought leaders in our space are having about trial design, about how we view the participants in the trial and how we care for them.

Even since 2016, the transition to partnership and recognition of the role participants and their families play in clinical research has made giant leaps forward, specifically around rare disease and recognizing the patient perspective and feedback in trial design and available support services. There are so many technology providers, and service providers like Scout that have risen to the top to make changes in how we conduct clinical research. I think we should all be very proud of the breakthroughs but also keep going, because we still have a very long road ahead. I love the discourse that we're having, and everybody's getting involved in the conversation: big pharma, biotech, the FDA. The breakthroughs are significant, and it's very exciting.

About KimberLee Heidmann
Scout co-founder KimberLee Heidmann has been with the company since 2001. She now serves as Executive Vice President of Patient Experience and Customer Success, as well as the Global Head of Quality and Regulatory Affairs. Her career in life sciences began in 1998 at a subsidiary of the World Health Organization Mental and Behavioral Disorders Team. KimberLee was part of the core team of innovators responsible for bringing Atlas Clinical Academy (now, Scout Academy) to market in 2018. Her passion lies in driving transformative change in the clinical research field, creating inclusive pathways to research care for patients regardless of factors such as location, economic status, age, disability, or race.

About Scout
Scout empowers the life sciences industry with people-first solutions: Scout Meetings, Scout Clinical, and Scout Academy. Since 1995, we have been a trusted partner in customer service, regulatory compliance, and project delivery for leading pharmaceutical and biotechnology companies. We specialize in face-to-face, virtual, and hybrid clinical meeting planning, patient payment, travel, and logistics support, and virtual collaboration and education. Our deep understanding of international regulation and adaptable network of resources is built on operational experience in 108 countries. With white-glove attention to detail and a customizable, comprehensive range of services, Scout makes the complex easier.

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