CONNECTING WITH RARE DISEASE COMMUNITIES: PATIENT ADVOCATES

Best Practices for Contract Research Organizations and Sponsors

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The biopharmaceutical industry is increasingly incorporating the voice of the patient in the drug development process, but how effective are these interactions from the perspective of rare disease patients?

To explore this question, we recently spoke to four leaders who have established their own patient advocacy groups and foundations. We wanted to hear about their unique experiences – both as people living with rare diseases and trailblazers for patient advocacy – to gather practical recommendations for the drug development industry. This article focuses on how to strengthen the connections between rare disease communities and a network of stakeholders, specifically examining how to best create mutually beneficial relationships with contract research organizations (CROs).

Understanding the impact of unmet medical needs

In order to work effectively with patient advocacy groups, it is important to understand the journey that prompts rare disease patients to step up and speak out. Many patients face significant challenges beyond dealing with the medical impact of living with a rare disease. For example, before receiving an accurate diagnosis they may have visited several physicians and specialists[1]. Their disease may have been unrecognized or overlooked, delaying necessary treatment.

Once diagnosed, the prognosis may vary widely, with limited or even no treatments available. Rare disease patients often have to fight misconceptions and can feel neglected by the medical community, and they routinely seek additional information about their disease beyond the boundaries of their healthcare providers.

Sentiments on the patient experience

Nadia Bodkin	Jew-EL Darboné	Shamonica Wiggins	Andra Stratton
"When most of us are first diagnosed with a rare disease, we have lost hope in the healthcare system. You realize how on your own you are and that doctors don't really know as much as you thought they did."	"Some rare diseases are invisible illnesses – you can't see when someone is in acute pain. It is frustrating when doctors don't believe your pain is real or think you are an addict."	"I've been misdiagnosed and experienced unfair treatment in the ER. A lot of patients in this situation just give up and do not get the treatment they need."	"Even with my background in research, I wasn't able to find any useful information about my rare disease online at the time of my diagnosis."

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The motivations behind the Rare Advocacy Movement (RAM)

Recognizing that these experiences are typical for people living with rare conditions, many patients and caregivers find solace in joining, or even creating, communities centered around common disease-specific goals. Although patient advocacy groups take various forms, most are dedicated to sharing resources, raising awareness about the rare condition, empowering patients to make informed decisions and advancing research.

The Rare Advocacy Movement (RAM)[2] was formed to evaluate and document the current rare disease patient advocacy landscape, uniting morally-vetted advocacy leaders with other rare disease stakeholders. Working together, RAM hopes to document the unique perspectives of each stakeholder and develop shared goals beyond bringing safe and effective rare disease treatments to the market.

Rare Disease Network of Stakeholders for the Rare Advocacy Movement

1. The Patient Community
2. The Caregiver Community (Parents & Non-Parents)
3. Biopharma/biotech Companies
4. Clinical Research Organizations (CROs)
5. Insurance Companies
6. Researchers/Scientists
7. Academics/Academia
8. Healthcare Providers
9. Regulatory Agencies

Lessons learned from industry experiences

In collaboration with RAM, we listened to four patient advocacy leaders as they shared their experiences interacting with CROs and biopharma/biotech companies. Based on their observations and insights, we have summarized the main themes of their conversations to create preliminary, working guidance on how CROs can best meet the needs of rare disease patients.

1. Rare disease advocacy groups want to be treated as respected partners

Patient advocacy groups should not be viewed only as intermediaries between industry and patients. These groups want to be involved in reviewing questions for the patient/caregiver community, providing input on protocols, understanding the expectations of the study, and helping to solicit feedback from their members.

Ideally, patient advocacy groups want to work in tandem with CROs to determine how a study can best incorporate patient-centric practices. As one advocate noted, “CROs can utilize the patient access that we’ve already established. We are very open to sharing information within our own networks.”

2. Patient advocacy groups are protective of their members

While many groups are eager to enhance patient-focused drug development, they also take pride in protecting their community members. Working with or providing funding to an advocacy group does not result in instant and unfettered access to the members.

Advocacy groups need to see evidence that an organization seeking access to their community truly cares about the patient perspective before encouraging their members to participate in an event, survey or study.

3. People with rare diseases appreciate extra support when invited to attend events

While patient advocates with rare diseases are honored when asked to attend an event or participate in a conference, the event sponsor should recognize that the patient advocates are providing a valuable service with their attendance. Traveling to a conference can be a time-consuming and exhausting trip. Patients appreciate when an event sponsor proactively asks about how to best support their trip, takes care of last minute details, makes special accommodations to address their needs, and promptly compensates them for their time and travel expenses.

During the event, patient advocates recommend considering any time zone differences for participants, building in sufficient break time for resting or maintaining a treatment regimen – and even limiting the amount of travel required, if visiting multiple venues within one conference.

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Participants also value the availability of healthy snacks compatible with dietary needs, comfortable chairs for travel-weary bodies, as well as pre-planning to address any potential accessibility issues with the venues. While these requests may sound extravagant to experienced traveling professionals, they are crucial to the well-being of the patient and can contribute to the success of the meeting or event.

4. People with rare disease want their input to count

When a patient with a rare disease, or their caregiver or family member, agrees to support a CRO's efforts, their viewpoint needs to be respected. No one else knows their unique rare disease experience or should speak to their perspective. CROs that show empathy and a willingness to listen can make a positive impression in the minds of advocates and their community members.

Patients and patient advocates also appreciate knowing how their information will be put to use: for example, to improve treatment practices, inform study design decisions or create greater awareness of the disease.

Sentiments on the patient experience

Nadia Bodkin
"Stakeholders have to understand the patient advocacy experience and landscape in order to truly be able to help us."

Jew-EL Darboné
"Listening to the patient is important to know what we are going through. We have a lot of knowledge and expertise – we have had this disease for all our lives."

Shamonica Wiggins
"Pharma companies often underestimate the amount of knowledge that is held within the patient population. People seem shocked and amazed that we know as much as we do about our disease."

Andra Stratton
"Patients are so eager to share their voice when someone can listen."

Opportunities for CROs to establish strong relationships with patient advocacy groups

Based on these insights from advocacy leaders, CROs and the pharmaceutical industry in general can consider several best practices to create more robust, patient-focused drug development processes.

1. Showcase a sincere commitment to the cause

Beyond publishing educational content or creating awareness through social media, the patient advocacy leaders recommend that CROs show their dedication to the rare disease research by being present. Patient advocacy groups appreciate when a CRO is sharing its thought leadership, sending its experts to conferences to discuss the latest insights, and even joining the conversation at patient advocacy meetings.

"We notice when companies attend our events and stay for the entire time," one advocate said. "We see that they are willing to learn about our rare disease and it makes us eager to work with them."

2. Involve patient advocacy groups in the protocol design process

A CRO can engage patient advocacy groups to gather input about a protocol, identify unrealistic expectations and ensure that they consider possible endpoints that matter to the patients.

One patient advocacy leader mentioned an example of a treatment that was being examined for its impact on triglyceride levels where a surprising positive effect on a symptom of the disease was observed during the study. Unfortunately, because the sponsor did not recognize the importance of this highly meaningful symptom to the patients, data concerning the symptom was not collected and the sponsor missed an opportunity to include the benefit in the product's submission documentation and label.

Rare disease patients are more willing to join a study if they are aware of potential benefits that better align with their ongoing health concerns. Finally, the availability of open access is also enticing, especially for those patients with poor treatment options.

3. Recognize and overcome intrinsic trust issues

Many rare disease patients have experienced sub-par treatment, misdiagnosis, and have even been denied treatments – factors that can contribute to a misperception of a "broken system" only looking for "guinea pigs" to test.

If a CRO creates visibility in the community and remains consistent in its messaging, patient advocacy groups and their community members will pay attention. CROs need to prove that they are an organization that supports patient-centered design and can help drive progress to treat rare conditions.

4. Ensure clarity in communication

CROs should pursue a policy of clear, open and honest communication, and work to increase transparency in their processes as a means of building trust. For example, with patient surveys, a CRO should provide as much information as possible about why the data are being collected and how these efforts will help reach established goals. Sharing the results with the community, to the extent possible, will increase willingness to promote a survey.

With research studies, it is important to explain how compliance, or the lack thereof, may affect the end results, and to provide clear, operational details on how compensation for study participation is dispersed. Patients should be informed as to when the results of the study will be accessible. Lastly, beyond monetary considerations, patients also appreciate a simple "thank you" for the contribution of their time.

Leveraging our unique position as a CRO

Covance and Chiltern, a Covance company, support sponsors through comprehensive capabilities that span the entire development and product lifecycle from pre-clinical to clinical and post-marketing. With this wide range of solutions, many opportunities exist to work directly with advocacy groups and integrate the voice of the patient throughout the drug development process.

However, as we learned when developing this article, gathering input from patients with rare diseases involves more than sending out blanket survey requests or consulting with a rare disease advocate. As a CRO, we must demonstrate our ongoing commitment to advancing research in rare diseases and recognize the role of patient advocacy groups as trusted partners with valuable insights.

As the focus on patients' perspectives continues to gain more attention, our industry can play an instrumental role in shaping constructive partnerships with rare disease patient advocacy groups. We must work closely with patient advocacy groups to determine how best to collect data and apply multiple perspectives that inform design decisions. These efforts can take place in tandem with ongoing discussions to solicit feedback and develop best practices on how to maintain effective collaborations between our teams and patient advocacy groups.

Not only will these joint efforts improve recruitment, compliance and retention in a study, they will help researchers and healthcare providers better understand a patient's needs beyond the clinic – and bring needed medicines and an improved quality of life to people with rare conditions.

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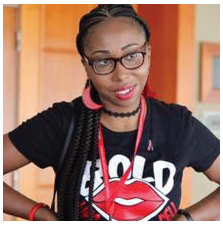

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About RAM: The Rare Advocacy Movement (RAM) is a patient advocacy initiative focused on documenting the ever evolving complex structure and unique dynamics of the rare disease patient advocacy landscape. RAM is a cultural awakening initiated by seasoned advocacy leaders that have vowed to remain transparent, clarify misunderstandings and ensure that the rare disease community is not overlooked, ignored or misrepresented. Learn more at https://www.rareadvocacymovement.com