Optimizing the Rare Disease Patient Experience

Recruiting for clinical trials is notoriously difficult. Inclusion and exclusion criteria are stringent, sites may not be convenient for patients, and the time required to participate can be a challenge too. For rare disease patients interested in enrolling in a clinical trial, these challenges multiply significantly. Read on to learn more about what rare disease patients want from the clinical trial experience, and how studies can be optimized to meet their needs.

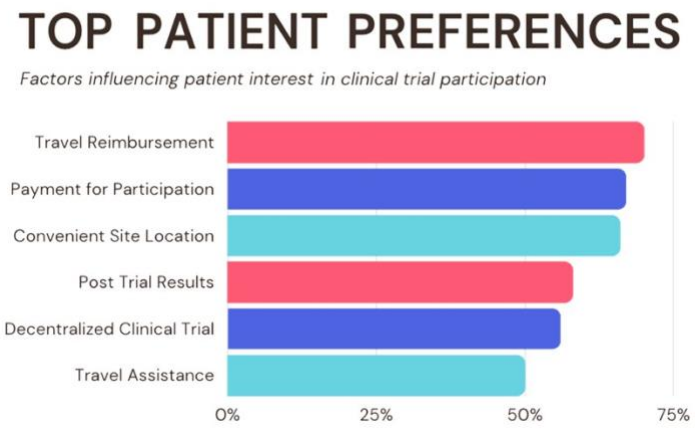
Rare Diseases and Clinical Trials

In the U.S., rare diseases are defined as those impacting fewer than 200,000 people. Over 10,000 rare diseases have been identified, impacting 30 million people—or, about 1 in 10.

Many clinical trials are being conducted in the rare space, but filling these studies is very hard. Some challenges to recruitment and retention include prospective participants' awareness of trials, any concerns and reservations they may have, and logistical obstacles to participation.

Rare Disease Participant Concerns and Needs

In a study conducted by Rare Patient Voice, 1989 patients and caregivers were asked about their preferences regarding clinical trials. Several areas emerged of great interest including risks versus benefits, a need for communication throughout the trial, the trial's location, and preferences for compensation and/or reimbursement. Patients were able to identify specific factors that would make participating in a trial extremely appealing to them, which are represented in the following chart.



Many patients identified concerns with trials including fear of the unknown, potential side effects, and whether participating might make their condition worse. They wonder about the effect the trial may have on daily life, the length of the trial, the potential risks of participating in a clinical trial, and the benefits compared to those of their current treatment.

Patients also want improved communication throughout the trial. They want to be fully informed of trial details prior to screening, including what the trial hopes to discover. Patients and family caregivers also want a 24/7 contact for emergencies or concerns, and an app or platform they can use during the trial. Finally, they would like to be able to connect with other people in the trial.

Improving Recruitment and Retention Through Engagement

Given the concerns identified as well as the suggestions for making trials more appealing, there are several things that sponsors can do to improve recruitment and retention—and they all start with engagement.

First, patients should be included in discussions around protocol design at the earliest stage. They know what they can reasonably bear and what could be too much to handle. Lay out the trial's goals and expectations up front (and in accessible language) so patients can help sponsors determine what makes sense for this patient group and how it will be perceived by the community. Planning ahead and engaging patients early in the process will eliminate many barriers.

Early engagement can also help sponsors consider implementing tools and processes which they may not have considered before, such as an app or a study platform for patients and their caregivers. Establishing a plan for reimbursement and a point of contact to help with travel arrangements will go a long way to help patients feel comfortable, and offering options for on-site as well as decentralized care will address the needs of those who prefer one over the other.

Conclusion

Rare disease patients may have never heard of the condition they have been diagnosed with, and their doctor might not be familiar with it either. There may be no available treatments, nor anyone working on one. Rare disease patients and families band together as a community to gain some traction toward finding a treatment or cure–if not for them, then for those who will be diagnosed in the future.

The rare disease community is very active, engaged, and connected out of necessity. They want to be involved and make a difference. Let's make clinical trial participation a little easier for them!

Scout offers a customizable range of patient-centric services in clinical trial travel, reimbursement, and regulatory support. If you're looking to tailor your protocol to meet the needs of rare disease patients and their families, Scout makes it easier. Visit https://www.scoutclinical.com/services *to learn more.*

About the Author

Pam Cusick, Senior Vice President at Rare Patient Voice, is an experienced research professional with more than 30 years of expertise in study design, implementation, and analysis. Pam earned a BA in Psychology from Sweet Briar College and an MA in Psychology from the New School for Social Research. She is Past-President of the Board of Directors and Scientific Advisory Council Lead for the Horses and Humans Research Foundation.

As Senior Vice President of RPV, Pam's focus is on the organization's continued growth and success, with an eye on client services, business development, and oversight of outreach and panel management, marketing, and human resources. She hopes to expand RPV's panels to include all patients and caregivers who want to share their opinions and impact their disease category.