Expand Your Circle – A Solution For Recruiting And Retaining Rare Disease Patients

By **Todd McGrath,**
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Globally, millions of patients suffer from over 7,000 rare diseases with few or no regulated standards of care. Even some well-known diseases, like cystic fibrosis, are considered rare diseases because they involve a wide range of genetic variability that affects their treatment. Yet, clinical trials often fail to meet recruitment requirements because not enough patients can reach clinical trial sites. Recently, Medical Research Network (MRN) analyzed data from 318 patients, over half of whom were engaged in rare disease trials, and found that the average distance from clinical sites was 177 miles in the United States and 136 miles in the rest of the world. For rare disease patients and their families, driving hundreds of miles round trip for clinical trial appointments isn't feasible.

Rare disease patients also face other challenges that make it hard to participate in trials. They may have severe physical limitations due to the disease and rely on caregivers for transportation, which limits their availability to travel. Sponsors wishing to study rare diseases must focus on building patient-centric trials as the best way to recruit patients and make it as easy as possible for them to participate. In-home clinical visits offer a patient-centered solution to recruiting and retaining rare disease patients.

Patients And Caregivers Want To Participate

Rare diseases affect the entire lives of patients and their families. Managing the disease's symptoms can be a full-time job. When patients participate in a clinical trial, it means they must navigate a complex web of doctors' appointments, treatments, and care. Despite these challenges, an NIH study in June of 2022[1] found that rare disease patients and their families are eager to participate in research studies to find new treatments. However, traditional study designs make it difficult for them to get involved. Also, study protocols have become increasingly complex in recent years, requiring additional tests, adding to the burden placed on patients.

MRN recently helped execute a trial for Duchenne Muscular Dystrophy, a rare genetic disorder that creates extreme muscle weakness and cognitive effects. Symptoms begin in early childhood and are frequently debilitating. As the child's mobility declines, it becomes increasingly more difficult for caregivers to take them to clinical trial sites for studies. When MRN worked with these families through our network of home healthcare providers (HCPs), we discovered despite their struggles, they were excited to participate in treatments that could help future generations affected by this disease. Many patients and their families who cope with rare diseases share this altruistic desire to advance therapeutics.

MRN bring a predetermined percentage of trial visits to the patient's home or an alternate location, such as a local clinic or school, to reduce the burden placed on patients and caregivers and give them more options to participate. In-home visits also mean less time missed from work or school and traveling long distances to sites, and more time in comfortable, often customized spaces in their own homes.

Increase Engagement And Reduce Dropout

Successful in-home visits hinge on positive relationships, beginning with the HCP and the patients and families they interact with. HCPs are trained healthcare professionals, often nurses, specially selected for each trial according to its needs and required proficiencies. They meet with patients to perform some of the study's protocol requirements, such as checking vitals, taking and processing samples, or administering investigational products. Often, when our HCPs deliver care in the patient's home or community, they establish trust and develop relationships with the patient and their caregivers, similar to what patients and families experience on-site.

"Patients with rare diseases and cancers must navigate a minefield of medical professionals and juggle a lot of appointments," explained one of our in-home specialists. "Being able to schedule a visit at home, have the IMP delivered, and have samples taken at home means that they can take a break from this. They can have their caregivers and family present, which also helps settle worries. A patient I cared for in their home had end-stage cancer, the monitoring was once a week, and she would always have her daughter there for comfort. I built a rapport with both and provided them with some welcome relief from hospital visits."

Additionally, trials looking to increase diversity can utilize in-home HCPs who are culturally sensitive, understand the community, and speak the patient's language. Visiting patients who are traveling is also possible so their participation doesn't pause because they are out of town. In-home visits can reach people where they are, anywhere around the world. This keeps patients engaged in the trial, allowing the trial to fit around their schedule, not the other way around.

Unfortunately, clinical trials have high dropout rates, which delays study completion. Patients may have adverse effects or miss appointments at the site. Or, they may struggle with self-administering the therapeutic or reporting their data correctly. Dropout rates are especially problematic for rare disease studies, given the protocol complexities and busy schedule of events. High dropout rates endanger the successful completion of the study, and thus prevent medically enhancing therapeutics from becoming a standard of care. Patient-centric trials that deploy HCPs to patients' homes can help ease the burden of participation so when eligible patients are identified, they have an easier time staying in the study.

An in-home HCP's role can cover almost anything in the protocol if the equipment can be carried into the home and is appropriate to conduct in the home, given the regulations in each country. MRN's in-home HCPs are enabled by digital technology so if a protocol requires a telemedicine visit, our in-home clinical professionals can facilitate them with the patient. Seeing a healthcare provider in person instigates a more meaningful connection than the patient calling the site to report their drug administration or adverse effects. It also allows the HCP to flag any symptoms or concerns the PI can follow up on to ensure the patient can successfully continue with the trial.

Ensuring In-home Visit Quality

Successful in-home visit implementation begins with the HCPs. At MRN, HCPs undergo rigorous qualification screenings unique to the clinical trial's requirements. First, HCPs are fully vetted nurses who meet national, regional, and local licensing requirements. Second, they must be experienced in the specific procedures and protocols that the trial demands. For example, if the study involves pediatric patients, the HCP must be experienced in working with children and their families.

Likewise, if the trial involves certain technical tasks, MRN requires that the HCPs have experience and training for that task. Next, our HCPs undergo extensive training followed by testing and interviews. The study's PI selects the HCPs they feel best fit the trial. Once in the field, we have an internal clinical services group and research lead that reviews the HCPs' documentation and ensures they follow all visit protocols.

MRN also uses a dedicated eSource platform that provides real-time data capture and validation and a steady workflow for the HCP, guiding them through all the data points in the correct order. This platform results in fewer data queries and reduces the labor needed to record and validate patient data.

Finally, in-home visits have complex logistical needs. Therapeutics may need to be stored at exact temperatures and delivered quickly. Patient samples also need medical storage, fast shipping, and meticulous documentation. MRN partners with couriers specializing in medical shipping. They can deliver or pick up materials precisely when needed, hold them at the correct temperatures, document any temperature changes, and provide a complete chain of custody.

MRN's Experience In Rare Disease Trials

MRN has partnered with sponsors to support over 140 rare disease trials and over 48,000 in-home visits for this specific patient population. In the past five years, more than 12% of our home visits have taken place outside of regular business hours at times that were more convenient for patients and their families, further meeting their participation needs. We work closely with sponsors, sites, and patients to bring clinical trials to patients' homes and communities and accelerate the drug development process.

About The Author

Todd McGrath is the Chief Operating Officer for Medical Research Network. He has 18 years' experience in the clinical trial and life science industry; beginning his career in pharmaceutical and biotech consulting and then holding operational positions at Parexel managing teams and projects.

Today, Todd McGrath oversees all the global operations for MRN. He has been instrumental to MRN's pharma clients, guiding them through operational challenges finding ways to maximize their results. His passion for making trials more accessible and less burdensome for patients and their families continues to drive MRN's vision of allowing more people to participate in trials and getting therapeutics to the market and patients faster.

About Medical Research Network

MRN accelerates patient recruitment and improves patient engagement and retention through site-centric and patient-centric solutions.

As an innovative market-leader, MRN provides customized solutions to optimize each individual protocol and creates more flexible, efficient and accessible clinical trials that deliver accelerated timelines. Through integrated in-home visit delivery and a vast global network of trained, research ready sites, all empowered by MRN's digital solutions, MRN engages with and empowers diverse communities around the world to participate in and advance medical research.

1. What role can decentralized trial designs play in improving rare disease studies? - PMC (nih.gov)

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