Making Decentralized Clinical Trials for Rare Disease Patients Commonplace

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A Rare Disease Patient Is More Than A Health Condition

To fully understand the impact a rare disease has on an individual, it's important to view the patient and their family as embarking on a journey with many steps along the way. According to a leading nurse-advocate for rare diseases in the UK — and the mother of a child with a rare genetic condition — it took five years of searching for answers to her daughter's baffling symptoms before she finally got a diagnosis of Sotos syndrome, a genetic disorder that causes excessive growth. She acknowledges being "delighted" when she finally received the diagnosis because it was the "key to unlocking the gate" to better understanding her daughter's disease and what information, treatment, and support would be needed to assist her journey and enable her to live her fullest life.

Often, the challenge for many rare disease patients is finding that understanding and support. Given the frequent delays in diagnosing rare diseases, many patients and families may feel lost and frightened, especially if they haven't found the appropriate testing and treatment resources. Even after a definitive diagnosis, further testing is often needed, which can be highly disruptive to the daily life and well-being of both a pediatric and an adult patient.

While clinical trials can be a critical key in "unlocking the gate" to the next stages of ongoing care and treatment, understanding the journey of the rare disease patient and family can provide invaluable perspectives in the development of the trial protocol, as well as its implementation. The philosophy that rare disease patients are individuals with families and lives — and not subjects — should inform every aspect of the trial.

Decentralized Clinical Trials: Taking The Trial To The Patient

Having the option to be seen in a home setting,or a hybrid of a home and clinic setting, rather than strictly a clinic or medical center, has become a growing lifeline for clinical trial patients during a global pandemic. As many trial sites closed and studies lost their patients due to COVID-19, the drug development and CRO industry became increasingly aware of the benefits of decentralized clinical trials (DCTs). Properly planned and implemented, DCTs have been shown to greatly increase recruitment and improve retention rates, as well as keep trials on track safely and efficiently. Using an experienced vendor from an international clinical trial organization, such as MRN, that has expertise in community- focused clinical trials — including over 100 rare disease trials — can bring the trial to the patient by conducting research visits in the community supported by global healthcare providers (HCPs) and clinical research experts. DCTs should focus on supporting clinical research delivery by helping patients participate in trials while remaining in their communities or homes, wherever they live around the world, helping clinical trial sponsors give their patients the option for flexible trial visits. MRN has spent close to two decades advocating for a decentralized, patient-focused approach to clinical trials. While the industry in recent years has considered various decentralized patient-centric strategies, COVID-19 has brought their implementation to the forefront of clinical trial development.

Mounting A Successful Home Trial For Rare Disease Patients

The Role Of Home Trial Support

In the case of the rare disease patient and family, MRN's Home Trial Support (HTS) service can offer convenient, tailored care in the patient's home, reducing the overall travel burden of a trial and enabling patient and family participation that might otherwise be logistically difficult. This is especially beneficial if the clinical trial protocol requires very frequent visits — or the site has been closed due to the pandemic. HTS also gives a critical element of control back to the daily lives of these patients — many of whom have complex daily routines — while still having access to the latest interventions and clinical treatments in the home setting.

Designing The Protocol

Yet, protocols aren't always designed with those elements that are most important for the rare disease patient, their families, or journey. Understanding the impact of a rare disease on a patient and their family while the protocol is being developed can provide valuable insights into the best approach to the trial, particularly how to make the study more convenient for all participants involved and reduce the burden on the patient. Not considering decentralized solutions, like HTS, proactively during the study design, especially during the worst waves of the COVID-19 pandemic, may derail the trial, resulting in services having to be retrofitted to the study, which can be difficult and cause delays. In designing the protocol, it's critical that upfront conversations take place among the study sponsors, the sites, and the vendors involved in implementing the trial, with a focus on what is best for the patient — not just what is written into the protocol.

It is also critical to minimize the risks inherent in the process of onboarding decentralized trial solutions, which comprises a complex web of systems, people, and activities that need to interact as seamlessly as possible to be successful. HTS services, for example, can offer a range of capabilities to manage all these moving parts, such as providing highly qualified and trained healthcare professionals to perform visits, managing and coordinating communications with the principal investigator and site teams,over-seeing data collection and the shipment of samples, obtaining informed consent, as well as many other logistical and regulatory components that go into implementing a clinical trial.

Setting Expectations

The beginning of a successful relationship with decentralized solutions starts with educating the site team and the patient and setting expectations of the service. Providing as much information as possible about what the trial is delivering is critical. Often, parents of pediatric rare disease patients are faced with having to decide whether to put their child through the potential disruption of a clinical trial versus the benefits of increasing their knowledge about a treatment, diagnosis, or therapy that may prolong their son or daughter's life. Engaging parents and patients with this information at the onset of the trial can help to lessen the inconvenience of the trial and help sustain participant retention.

Routine and structure are also essential components for patients and families living with a rare disease. Therefore, changes in schedules and how they may affect the patient also need to be considered when undertaking home trials. In addition, patients and families must be prepared for new healthcare providers coming into the home. While the HCPs involved in the study may be new, it's important to reassure the patient and family that this will not disrupt their long-standing patient-physician relationships. Depending on the type of study, there may also be equipment involved. This could include a centrifuge, an infusion pump, blood collection materials, as well as flashing lights or beeping sounds. The family as a whole, including any "furry" members, should be prepared for these interventions. In addition, since an HCP may need to remain in the home longer than a typical clinic appointment, this may put a strain on the family's regular routine. In order for home trial support visits to be successful, it's imperative that the study details are covered in full, and expectations set at the onset, to ensure that everyone impacted by the clinical trial is comfortable and well-informed.

Supporting The Patient Journey

Whenever possible, providing patient-centered support and care for the patient as well as the family is critical. Whether the trial is conducted at the site, in the home or other decentralized location, it's imperative that trial schedules are flexible and meet the needs and lifestyles of the patient and the family members involved. This may include siblings, partners, and grandparents who often play a significant part in the care pathway. The trial design must also consider that patients are at various stages of their journeys. An adult who has been newly diagnosed with a rare condition may have very different information and support needs than a young child, or an individual who is at a later or end stage of their particular condition.

Yet, it's important that all these aspects of the patient journey be taken into consideration when planning and recruiting patients for a decentralized clinical trial. A newly diagnosed adult might be in a better

state of health and can engage more easily with their clinical teams at a site. For someone who is in the later stage of their condition, it might be more practical for them to be seen in a home environment. Parents of patients with young siblings may also have particular concerns regarding the impact on their other children, such as being brought along to clinical visits or seeing medical equipment and procedures performed inside their homes.

Regardless of the diagnosis, the trial design and planning should consider creative, flexible approaches for rare disease patients. First and foremost, patients are individuals who are part of a family unit and have lives outside of their clinical trial. To be successful, a decentralized clinical trial protocol requires expertise in trial design and implementation, sensitivity to the needs of rare disease patients, and a commitment to helping rare disease patients and families find answers to unlocking that next gateway in their journey.

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