Operationalizing Recruitment and Retention in Rare Disease Trials

Highlights:

- Rare disease drug development increased 41% between 2019 and 2020
- Developing relationships with patient advocacy groups and key opinion leaders is the foundation of a successful patient recruitment strategy
- To increase retention, implement patient services that reduce patient and family burden into rare disease protocols
- Strategic social media management helps you get your message out to patient communities

Recognize the Challenges

Between 25 and 30 million Americans and about 400 million people worldwide live with a rare disease. In the United States, a disease is considered rare when it affects less than 200,000 people. The European Union defines a disease as rare when it affects less than one in 2,000 people.

A better understanding of the genetic basis and pathophysiology of rare diseases, combined with increased support from the FDA and other regulatory agencies, have prompted a surge in rare disease drug development over the past 10 years. In 2020, the FDA approved 32 novel drugs and biologics with orphan drug designation: 31 pharmaceuticals and one biologic. The agency received 753 new requests for orphan drug designation in 2020 — a 41% increase from 2019.

With about 7,000 rare diseases identified, all with relatively small patient populations, and only 5% of which have FDA-approved treatments, drug developers have many more therapies to discover. Orphan drug development is not only the right thing to do for rare disease patients, who generally have a very high unmet need, it can also be a wise business decision.

Orphan drug designation qualifies sponsors for tax credits, Prescription Drug User Fee Act waivers, seven-year marketing exclusivity (plus six months for pediatric indications), and the ability to apply for research grants from the FDA and other entities, among other benefits.

However, finding eligible patients to participate in rare disease clinical trials is an ongoing challenge. Patients with a particular rare disease may be scattered across the globe, so a global approach to identifying and enrolling patients is particularly

important. Fifty percent of individuals affected by rare diseases are children, a fact which introduces challenges of its own.

To overcome these challenges, sponsors must develop a solid patient recruitment strategy. That strategy must take into account the unique needs of the patient population in question: where they live, how they live, and what they need to participate through the duration of the trial.

Customize Your Approach

Because no two rare disease populations are alike, each study requires a tailored approach to recruitment. As you develop your strategy, consider the following:

- Indication
- Characteristics of the patient population
- The burden of the trial on patients, caregivers, and families
- Patients' locations
- Site locations
- Active advocacy groups
- Location of key opinion leaders
- Marketing strategy: social media, advertising, or both

Establish and Nurture "Rare" Relationships

Before finalizing the protocol, connect with organizations and individuals that will help raise awareness and connect you to patients: advocacy groups, key opinion leaders (KOLs), and patient registry organizers. These entities can not only help you find patients, they can also advise on ways to make your protocol more attractive to patients and families.

Patient advocacy groups educate and support patients living with a particular disease, disability, or condition. Advocacy group leaders can inform their communities about your trial, allowing you to connect with a large segment of a rare population through one connection. Once you build a relationship, you can ask group leaders to disseminate recruitment information through social media, emails, newsletters, and even family meetings.

KOL physicians and centers of excellence (COE) are crucial for identifying patients. Few physicians have the specialized knowledge and expertise to properly treat rare disease patients. Due to the lengthy diagnostic odyssey for many rare diseases, patients often move from their primary care physician to one or more specialists without a diagnosis or treatment.

COEs provide care for patients suffering from a particular rare disease or group of diseases. Within COEs, you'll find KOLs who treat patients with your targeted rare disease. KOLs can help refer the right patients to your study. These centers and physicians could become sites and principal investigators, respectively.

KOLs, patient advocacy groups, health systems, and even patients and families develop **patient registries** and/or **natural history databases**. These registries help researchers, physicians, and patients better understand patient demographics, geographic distribution, and the clinical characteristics of the disease. They may also help drug sponsors identify potential subjects for clinical trials.

Recognizing the recruitment challenges in rare disease studies, the National Institutes of Health (NIH) funded the Rare Diseases Clinical Research Network (RDCRN) Contact Registry, which includes more than 200 rare diseases. Through the registry, patients can learn about currently recruiting research studies. It's worth a look.

Harness the Power of Social Media

Nearly 3.6 billion people worldwide have at least one social media account. In the United States, more than 40% of healthcare consumers turn to social media for healthcare information.

Given participant numbers and its global reach, social media can be a powerful tool for patient identification. But without a strategic plan, your outreach won't be as effective as it could be. To ensure potential rare disease study participants see your social media posts, consider the following steps:

- Define your audience's demographic and physical characteristics
- Identify your target audience's preferred social networks. For example, more than half (53.8%) of Facebook users are age 25 or older. Most (82%) Snapchat users are under age 34.
- Develop content individuals and organizations can share. In addition to articles and links to study websites, patient advocacy groups may share IRB/IEC-approved materials with their followers.
- Continue to engage with advocacy groups throughout the enrollment process. They can continue to share your content, which keeps you top of mind with their patient networks.

Develop Excellent Patient Service Practices

Patient centricity is crucial to attracting and retaining rare disease study participants. While many patients want to participate in clinical trials, not all of them can manage the demands on their time and energy.

Consider the following options to make patient convenience a top priority:

- **Travel concierge:** These companies handle logistics, accommodations, and travel arrangements for clinical trial participants, their families, and/or their caregivers.
- **Translations and Interpreters:** For global trials, offer translation and interpreter services for communication and documents. Some travel concierge companies offer this service.
- **Reimbursement:** Reimburse patients quickly for parking, meals, childcare, and other expenses. Swift reimbursement shows you are responsive to their needs.
- **Telehealth:** Virtual visits proved essential during the COVID-19 pandemic. Now that more patients feel comfortable with telehealth, implement this service when possible.

- **Home healthcare:** When an evaluation requires the hands of a clinician, consider sending nurses to patients' homes. Rather than travel to a site for a blood draw, the patient can provide the sample from the convenience of home.

Look for patient services vendors who can cater to your patient population, indication, and geographic location. Your CRO may also have established relationships with experienced vendors.

Rare Disease Trials Require a Patient-Centric Approach

As competition heats up in the rare disease space, a targeted patient recruitment strategy will help you identify and enroll patients as quickly as possible. As you develop your protocol, keep patient needs front and center. Partnering with vendors that understand the scientific, regulatory, and operational complexities of these valuable clinical trials will help align your program for success.

About Synteract

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