Considering the Patient Journey for Rare Disease Clinical Trials

Providing Information Online

Patients often seek out information for clinical trials on their own, and may be part of a robust online rare disease community.

23% of Internet users *with a rare disease seek out peers online.*

Shute N. People coping with rare disease are internet power users. NPR. https://www.npr.org/sections/health-shots/2011/03/04/134140813/people-coping-with-rare-disease-are-internet-power-users. Published March 1, 2011. Accessed February 28, 2022.

Supporting Patient Caregivers

Patients are often very ill or children, and require a caregiver who must also be "recruited" into a study.

50% of rare diseases *affect children.*

Anticipating and Reducing Logistical Barriers

Rare disease patients often face logistical barriers when considering clinical trial participation. This may include long travel, frequent clinical assessments, and incompatibility with job requirements.

According to a 2019 survey, ***17% of individuals noted had relocated or considered relocating*** *for access to medical care related to their rare disease. When it comes to clinical trials, Sponsors should work to give patients more flexible options to reduce logistical burden.*

Consider allowing patients to participate through ***partial or full remote access,*** *using tools such as:*

- *Telemedicine*
- *Home health visits*

NORD RareInsights. Barriers to Rare Disease Diagnosis, Care and Treatment in the US: A 30-Year COmparative Analysis. NORD RareInsights. Published 2020.

The Role of Patient Advocacy Organizations

Many patients will be involved in a patient advocacy organization (PAO) relevant to their disease. Researching & profiling PAOs can help sponsors determine the role they may be able to play in the study.

A 2016 survey found that ***PAOs contributed to over 40%*** *of the total number of patient enrolled in rare trials.*

Merkel PA, Manion M, Gopal-Srivastava R, et al. The partnership of patient advocacy groups and clinical investigators in the rare diseases clinical research network. Orphanet J Rare Dis. 2016;11(1):66. Published 2016 May 18. doi:10.1186/s13023-016-0445-8

A client-focused CRO with rare expertise

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Actionable Steps for a Successful, Patient-Centric Rare Disease Trial

Bring in Biostatisticians Early

As the potential patient pool is limited for rare disease studies, bringing in biostatisticians early can help ensure a more patient-centric design (for example, considering cross-over or 2:1 or 3:1 randomization).

Research & Profile Patient Advocacy Organizations (PAOs)

Early in study planning, project managers should work to determine the potential role a PAO can play, including what kind of support or insight they can provide.

Incorporate Decentralized Aspects

Work with vendors who can assist in bringing forth virtual trial options, such as leveraging telemedicine and home nurses.

Interview with Principal Investigators (PIs)

PIs may be able to recommend other PIs, advocacy groups, support groups, etc. that are relevant to your study indication.

Maintain an Online Presence

Consider creating an online presence for patients to find your study on their own. This may include leveraging social media or Google advertising, as well.

Work Closely with Site Staff on an Ongoing Basis

Leverage a site's unique knowledge across the entire study, sharing successes and lessons learned. Additional support - such as identifying potential events where the rare disease community may be coming together - may be helpful as well.

For more information, contact our team: pharpoint.com/contact