Published in February 2017
PharmaVOICE

SPEAKout

Provided by: Worldwide Clinical Trials

Maximizing Patient Recruitment in RARE DISEASE RESEARCH

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Creating a pharmaceutical development program for the treatment of a rare disease is challenging. From recruiting and retaining patients and investigators, through to regulatory uncertainties and limited understanding of the natural history of the proposed indication due to few observational trials studying disease progression, there are many considerations and hurdles to overcome.

Despite permissive science, innovative trial methodology, evolving regulatory sentiments and increasingly sophisticated commercialization all supporting rare disease drug development in recent years, patient recruitment can become one of the most daunting challenges in rare disease drug development.

Maximizing Participation

Sourcing and identifying participants for rare disease studies can be an obstacle for researchers due to smaller and geographically dispersed patient groups.

Small patient numbers mean that the involvement of every single patient is vitally important. It also means the impact that clinical endpoints from a limited number of patients may have on program development is exceptional. For this reason, engaging sites, investigators, and patients to confirm acceptance of the study design is vital. Proactively engaging all stakeholders can foster a collaborative approach that not only facilitates recruitment, but also retention and commercial value long-term.

Advocacy Outreach

Advocacy groups exist for most rare disease indications. Contract research organizations (CROs), sponsors and investigators should engage with these groups, especially during the initial stages of a protocol design, because having their cooperation can be crucial to the success of a trial. These groups usually include patient caregivers or relatives, as well as physicians and key opinion leaders (KOLs) in the specific indication. By taking the time to meet and speak with members of relevant groups, investigators can gain valuable insight on a particular condition, including what life is like for patients, as well as what is important to caregivers and others in the patient's network

Due to the rarity of some conditions, there may be no established advocacy or patient groups. In this case, general registries such as the Global Rare Disease Patient Registry and Data Repository, entities such as the National Organization for Rare Disorders and the European Organisation for Rare Diseases, as well as resources such as Orphanet, are invaluable as a first step in site identification and selection. Researchers can also make use of international rare disease research consortiums, such as Eurodisk and the National Center for Advancing Translational Sciences, which work to foster international collaboration through preclinical and clinical research in rare diseases.

Supporting Recruitment

In order to further support recruitment, it is also crucial for researchers to gain the trust and buy-in of patients and their caregivers. More so than with non-orphan conditions, sufferers of rare diseases, as well as their caregivers, are typically well informed about their condition and the latest research. Ultimately, if they do not buy-in to what the team is trying to achieve with the study, or the suggested approach, then there is little hope of recruiting the patient.

Researchers should bear in mind that physicians treating sufferers of rare diseases often have a strong relationship with their patient, which can sometimes result in a reluctance to enroll them in clinical trials at sites outside of their jurisdiction. For this reason, a physician who is fully engaged and informed about a study from the offset is more likely to share motivations and recommend to patients that they participate in the trial. Additionally, physicians and investigators specializing in rare disease are typically well connected on a global scale. Researchers should proactively reach out to the most influential in the relevant indication in order for their study to optimize patient accrual.

As with any trial, the whole research team must be fully engaged from the onset of the study and throughout in order to maximize patient recruitment. By involving the team in discussions and planning as soon as possible, sponsors can ensure everyone feels engaged and that they are adding value to the protocol, and as a result, are more likely to pass this enthusiasm and understanding along to potential participants.

Conclusion

While it is clear that creating a drug development program for the treatment of a rare disease is no easy task, there are a number of steps researchers can take, particularly when it comes to patient recruitment. By putting the patient and their care network at the heart of the study through careful integration of design and operations, and by engaging with influential KOLs, regulators, patients and their care network from the offset of the study, researchers can improve drug development efforts in this field.

For further information on strategies to recruit and retain rare disease patients, visit www.worldwide.com.