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Why we need to redefine the "team" in rare disease trials

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Achieving a sponsor's goals, though of utmost importance, should only be one area of emphasis during a clinical trial. One must also consider the broader team of stakeholders—the patients, their caregivers and their physicians. This is never more true than in the study of rare diseases.

By their very nature, rare conditions are not always well understood, and the endpoints for study success are not always well established. This, often combined with small, scattered patient populations and a high proportion of younger subjects, calls for well-thought-out protocols and teams that understand how to operationalize these challenging studies.

Patient recruitment for rare disease trials can be difficult, which makes the retention of both the patient and their care team critical. Investigators should work in tandem with patients, their caregivers, physicians and patient advocacy groups to consider each patient's individual experience. Rare diseases patients and their caregivers are typically well-informed about their conditions, meaning their insights should be considered in the study design; this helps foster a feeling of ownership in the outcome of the study on behalf of the subjects. Finally, clear communication of the study plan is important to both recruit and retain patients.

Understanding the patient experience during the design phase can ensure the intent of the trial is met. For example, in a study involving young Duchenne muscular dystrophy patients, one of the standard assessments was an early morning walk test. Through engagement with an advocacy group, it was found that children with this condition may suffer from stiffness in the morning and preform this assessment at a disadvantage, leading to less accurately representative results. A simple adjustment to the assessment schedule resulted in a better outcome.

Gathering input from as many stakeholders as possible during the clinical study design phase and embracing a larger and more inclusive team can be integral to success. By working together, these teams will be able to deliver outcomes for future treatments that are fully informed by those most eager to receive the benefit of these medical discoveries.

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