Strategies for Accelerating Rare Disease Clinical Development:
“Where Does Your Rare Disease Trial Fit?”

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Executive Summary

Rare disease trials differ from those in most other therapeutic areas because the indications are more complex and involve smaller patient populations. It is essential to understand the current disease landscape, the patient pathway, and the competitive clinical trial environment when positioning a new clinical trial within the rare disease space. Drug developers must be aware of the status of the indication and the location of open trials, their phases, and the patients they seek to enroll. Understanding the details of these trials is critical: Which are true competitors and which key opinion leaders (KOLs) should be engaged? Where does your trial fit? Is there so much competition that you may need to take your trial to another geographic area?

This white paper discusses strategies for accelerating rare disease clinical development by leveraging data in a more evidence-based approach to guide the design of a trial; to understand treatment trends; and to identify the best investigators, sites, and patients.

Introduction

Drug development in disease indications is increasingly complex. Rare disease clinical trials often involve small numbers of diverse patient populations, wide geographic distribution, and endpoints that may not be fully validated. Diagnostic criteria, the natural history of the disease, and the disease progress are poorly understood for many rare diseases. Knowing where your trial fits into the competitive landscape can mean the difference between success and failure.

Challenges for New Clinical Trials in Rare Diseases

Drug developers face a unique set of medical, scientific, ethical, and operational challenges when conducting clinical trials in rare disease indications. The primary concern is locating patients. Can enough patients be identified and recruited into a study?

Sites have traditionally been tasked with assessing these studies and developing recruitment plans based solely on their experience. In this competitive research environment, locating investigators capable of conducting clinical trials in rare indications and recruiting suitable patients requires a customized, data-driven approach based on a deep understanding of patients, the indication, and competing trials. Drug developers can use this knowledge to weed out the true competitors and align their study goals with programs that will support their development plan.

Positioning New Trials in the Rare Disease Space

It can be perplexing to determine how a program will fit into the rare disease space. Drug developers should leverage public, private, and proprietary data sources to drive trial design, identify the investigators who treat the most patients, and understand treatment trends. These strategies help drug developers position the trial effectively.

Understand the Indication & What Has Been Accomplished

As drug developers approach a rare disease program, it is important first to understand what has already been accomplished in the disease setting:

• What is known about the indication?
• Are treatments available?
• What are the experiences of the patients and caregivers who have this disease?
• What types of trials have been conducted and completed?
• Are endpoints and outcomes clearly established?

Completed trials can guide future development if we use their insights to guide study designs and endpoint selection, and to identify the best locations and sites. Look closely at similar completed studies to identify sites with strong enrollment that effectively moved therapies forward:

• How many sites and patients were enrolled in past trials?
• What were the eligibility criteria?
• What were the efficacy assessments?

Use this knowledge to determine how many sites will be needed and to develop a realistic accrual rate.

Assess the Competitive Landscape

Assess the status of the indication to see what research is underway:

• Where are open trials located?
• What phases?
• What are the target patient populations?
• What are the eligibility criteria?
• What are the durations and enrollment windows?
• What type of investigational product is being studied?
• How is the product administered?
• What do we know about the product’s safety profile?

Consider the distribution of studies and sites for open global trials and identify which countries have the most open studies. Use this information to develop insights into the most (and least) competitive, saturated, and active regions.

From the list of suitable regions, next determine where sites are open in specific countries. Look in places where there are expert centers to determine whether they have trials underway then determine which key investigators are interested in participating in a trial.

Compare Eligibility Criteria

Because the demand for patients is a key component of competitive trials, we must determine how saturated the clinical trial space is. The presence of several open trials can be an obstacle for new trials as they will compete for enrollment. However, depending on eligibility criteria and timelines, these trials may also serve as feeder studies to support and supplement recruitment.

Consider the specifics of the patient population. Compare the inclusion and exclusion criteria of existing and proposed trials. Determine whether an existing trial is truly a competitor, or whether the eligibility criteria are sufficiently different to complement the new trial and create a recruitment stream. Patients might also roll from one study into another.

After examining the details of the competitive landscape, drug developers will better understand:

• Where are open feeder trials?
• Are there any patient registries in place?
• Who are the experts and which KOLs should be engaged?
• Which investigators have the most experience?
• Which investigators can help champion their trial?
• Is there an established research network or consortium?

This knowledge helps drug developers determine the best location for the trial.

To Ensure the Attractiveness of a Trial, Consider the Barriers

In addition to the location and patient populations of ongoing trials, drug developers must also consider the range of therapies in development. These vary with respect to convenience and attractiveness for patients. For example, some therapies may involve oral medication that children can take at home versus an intravenous (IV) therapy that requires frequent travel to the site. Gene therapy is often a one-time administration, while some new biologics may require treatment for year or longer. When designing the trial, drug developers should take into account common barriers and make participation as convenient and easy as possible for patients, caregivers, and families.
Understand Treatment Trends & Plan Accordingly

Treatment trends can have a major impact on a trial’s position in the rare disease space. To position a study optimally, drug developers should identify these trends. It is important that drug developers understand existing treatment trends with regard to how they relate to a trial’s clinical development. Understanding how treatment paradigms are changing is particularly important when considering combination-therapy studies or work in second-line settings. If it takes years to conduct a trial and get results, drug developers must position a comparator or benchmark that will be relevant when the study is completed.

Summary & Recommendations

It is essential to apply our understanding of the current clinical trial landscape and competitive environment when positioning new studies in rare disease space. Strategic placement of a clinical trial in rare indications requires thorough planning. To understand where their trial fits best, drug developers must understand where open studies are located, which are true competitors, and which could serve as feeder studies for a new trial. Furthermore, leveraging data in an evidence-based model to identify sites and investigators with proven records in rare disease enrollment will help drive a clinical program to facilitate faster patient accrual.
Contact Information

Do you know how your trial fits into the rare disease space? If not, it may be time to look for a trusted partner with experience and expertise managing studies in rare diseases.

For further information about PRA’s service offerings or to schedule a complimentary consult with our rare disease expert, please contact Scott Schliebner, Vice President, Scientific Affairs–Rare Diseases at PRA.

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