PREPPING FOR SUCCESS: FIVE OPERATIONAL CONSIDERATIONS FOR RARE DISEASE PROGRAMS

BY JEFF O'HAGAN, ASSOCIATE DIRECTOR, GLOBAL PROJECT MANAGEMENT
WORLDWIDE CLINICAL TRIALS

As a veteran project manager, I've spent the majority of my career in operations management, either on the part of the sponsor or on the part of the CRO. I've provided expertise or oversight in a number of therapeutic disciplines ranging from cardiovascular to infectious disease to oncology to pain. I've participated and managed highly complex projects, such as an oncology program involving five different treatment arms, a stepwise approach for dose increases for maximum tolerability, tedious cohort assignment/management in phase 1 studies, high incidental SAE reporting, and delicate and intricate IP preparation scenarios requiring compounding for infusions, to name a few.

Despite my extensive operational expertise and experience, nothing could prepare me for the complexity of my first rare disease program, studying spinal muscular atrophy (SMA) in young pediatric populations. This project required that I draw upon all my understanding of complex study design, operational delays in study start-up, retention of patients, and, equally important, family engagement. And, all my previous expertise notwithstanding, I came away from the SMA study with five new learnings for managing a rare disease study:

GET PERSONAL WITH YOUR PATIENTS.

Rare diseases affect small populations of patients, their family members, other caregivers, and the extended "family" of other patients with the condition. In rare disease studies, recruitment numbers are secondary to the individual patient experience, so each patient is a study within a study.

PATIENT ADVOCACY RELATIONS ARE ESSENTIAL.

Particularly in the case of rare diseases, these individuals are your experts on the condition and your influencers with patients. By reaching out to patient advocates and visiting them in person to introduce your project and its protocol, you build a level of trust that's worth your investment of time and finances. Seek their input and support on recruitment and retention strategies, and keep in contact with them throughout the course of your project to maintain a relationship of mutual support. Partnering with advocates early in your process, even during pre-startup, can mean the difference between make and break for your trial.

VENDOR MANAGEMENT TAKES HIGH PRIORITY.

For the spinal muscular atrophy study, we engaged 32 vendors during the course of the program, including one who provided Geoffrey the Giraffe toys to be shipped to 18 different countries. Rare disease studies call for highly specialized medical vendors, specific to the exact data points required to assess the unique patient condition. Logistical expertise becomes paramount as you look beyond the traditional and well-known companies, like ERT, BioClinica, or LabCorp.

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BE EXTRA SELECTIVE AS YOU BUILD YOUR STUDY TEAM.

Rare disease programs challenge the status quo and call on all team members to check their professional ego and motivations. As a foundation, PMs, associates, and CRAs must be knowledgeable in the specific rare disease area. But beyond expertise, team members should have a willingness to take the extra time and a drive to push forward a cause that may not result in a large financial return for the company. Success in a rare disease study depends on study team members who are passionate about helping these patients.

WORK FROM BOTH YOUR HEAD AND YOUR HEART.

Each rare disease patient has their own disease history. Remain cognizant of the fact that day-to-day living for many rare disease study subjects is already challenging, even before they undertake the added responsibilities of completing the required study assessments and maintaining investigator appointments. Successful patient retention and compliance in a rare disease study demands that you engage your empathy alongside your operational expertise because the best way to optimize the program is to keep the best interests of your patients and families in mind.

At Worldwide Clinical Trials, we pride ourselves on our uncommon approach to clinical trials. For my part, I can say that my work on the SMA program challenged me to think in new ways and reignited my passion for helping patients.

ABOUT JEFF O'HAGAN

Jeff O'Hagan is an Associate Director, Global Project Management in Neuroscience, with more than 23 years of experience in the biopharmaceutical industry, including over 10 years in international clinical operations leadership roles. Jeff has strong strategic, organizational, and interpersonal skills, as well as an excellent track record of building highly effective cross-functional teams and collaborations, including with patient advocacy groups and research associations. Jeff's expertise in clinical trial development spans the full development life cycle, from first-in-human to late phase clinical trials and registry studies. He has experience overseeing and leading global teams and studies conducted across Asia-Pacific, Europe, North America, Latin America, and the Middle East. He has experience in mentoring, line-management, training, co-monitoring, and quality assurance audits. Jeff's employment has been equal parts on the sponsor side managing global CROs as well as client-facing capacities within CROs. Jeff holds a BA majoring in pre-medicine/biology, minoring in psychology, from Lehigh University in Bethlehem, Pennsylvania. He is currently home-based in Los Angeles, California.