Rare Disease: Trial Strategies and Solutions

UBC offers innovative solutions to pharmaceutical and biotech sponsors to reach recruitment and retention goals for Phase II-IV rare disease trials. This includes access to data via pharmacy and medical claims, customized site support, and cutting-edge technologies. This, coupled with our vast rare disease trial experience and successful relationships with rare disease advocacy groups and online community experts, differentiates UBC in the patient recruitment field. We look forward to bringing these strengths to your programs, making your clinical trials efficient, and delivering on time.

Keys to Recruitment and Retention Success

Tap into Advocacy Groups

When it comes to clinical research for rare and orphan diseases, the relationship with patient and advocacy groups is especially important. Advocacy groups for rare diseases are actively seeking information, especially for areas without a cure.

Customization is Key

Cookie cutter programs do not work in the rare disease space. We customize everything related to a rare disease study because it gets results — increased site engagement, more enrollments, and patients who stay engaged throughout.

Patient Centricity is not Just a Buzzword

When patient populations are small, it's important to retain each study participant. We take the time to understand the patients in a rare disease study. This allows us to offer services, like home health nurses or concierge travel options, that make participation easy.

Thought Leadership: Insights from the Team

- Working with Advocacy Groups: Making it a Win-Win Collaboration
- Rare Disease Day: Driving Progress Through Collaboration
- Making Rare Diseases Voices Heard
- Takeaways from the NORD Rare Summit: Zebras Among Us
- Confronting the Challenges of Rare Disease

Rare Disease Experience Includes:

- Achondroplasia
- Acromegaly
- Acute Lymphoblastic Leukemia
- Alpha-1 Antitrypsin Deficiency
- Anemia in Pediatric Chronic Kidney Disease
- Chronic Myeloid Leukemia
- Crohn's Disease
- Cushing's Disease
- Cystic Fibrosis
- Fabry Disease
- Factor XIII Deficiency
- Gaucher Disease
- Huntington's Disease
- Idiopathic Pulmonary Fibrosis
- Immune Idiopathic Thrombocytopenia
- Juvenile Idiopathic Arthritis
- Lysosomal Acid Lipase Deficiency
- Multiple Myeloma
- Myelodysplastic Syndrome
- Acute Myeloid Leukemia
- Niemann-Pick Disease
- Phenylketonuria
- Pompe Disease
- Pulmonary Arterial Hypertension
- Pyoderma Gangrenosum
- Renal Cell Carcinoma
- Short Bowel Syndrome
- Spinal Muscular Atrophy
- Urea Cycle Disorders
- Uvetis
- Myelofibrosis
- Neuroblastoma

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Case Study: Utilization of Clinical Trial Nursing: Rare Disease Trial

Challenge

UBC received a phone call from a sponsor who reported they enrolled their first subject in the placebo arm of their Phase III rare disease trial. The subject experienced immobility and was unable to complete any of their activities of daily living (ADLs). The subject's caregiver was struggling to provide care for the patient. The subject was ready to withdraw from the study; desperate to stabilize symptoms by resuming the open label drug via the separate trial run concurrently by the sponsor.

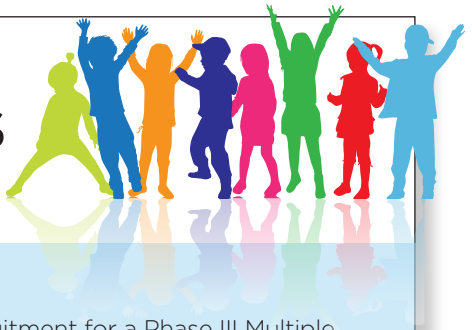
Intervention

UBC identified nursing agencies within the UBC network that could provide 24/7 health care services, for the subject for the duration of their stay. UBC staffed a private duty aide within 45 minutes of the initial phone call from the sponsor.

Outcome

The patient was able to receive the care that was needed to remain on placebo, and completed the remaining study requirements. The sponsor did not have to extend the recruitment period, and overall study time lines were not impacted. More importantly, the patient and caregiver needs were addressed, and the intervention positively affected their experience in participating in the trial.

Case Study: Pediatric MS Recruitment



Challenge

UBC supported patient recruitment for a Phase III Multiple Sclerosis Study for children aged 10-17.

- The study had a 60% screen fail rate, and included weekly injections for both placebo and treatment arms.
- Many of the centers enrolled in the study were located far from the patient population.

UBC Solution

- The biggest support success was a global transportation program that allowed children and caregivers to attend study visits without extra burden.
- With this support across numerous countries, the study exceeded enrollment goals. UBC also provided sites with educational materials, online outreach and advocacy support.

UBC Difference:

Meeting the specific needs of rare disease study participants based on challenging trial needs is key to UBC's approach to patient recruitment.

Shazia Ahmad

Director, Patient and Physician Services

Ms. Ahmad has cultivated strong relationships over the past several years with key organizations in the rare disease space. This has included regular speaking collaborations on relevant topics in the rare disease community. Shazia provides oversight and strategy on patient recruitment and retention as well as stakeholder engagement. She previously served as a study coordinator at the National Institutes of Health (NIH) and has a rich understanding of patients with a rare disease.

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