CTI CLINICAL TRIAL & CONSULTING

Newsletter

Where Life-changing Therapies Turn First®

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CTI Pushes Life-changing Therapies Forward in Rare and Under Served Populations

CTI is a leader in rare disease and orphan disease research with approximately 80% of our work falling into this category. Successful rare disease /device development programs require experience, expertise, and dedication. They are often fraught with challenges from designing an executable protocol to patient and investigator identification. As a leader in rare disease research, CTI has the experience required and the resources necessary to support drug/device development in this complex area from the regulatory planning stage through marketing approval.

Protocol/Study Design

The first step to a successful rare disease trial is the development of an executable protocol that is as simplified as possible while still maintaining the integrity and objectives of the study and overall development program. A comprehensive understanding of the disease is key to developing a sound protocol. Understanding prevalence/incidence of the disease, studying care pathways, examining patient clustering and looking at any existing data are all critical factors in obtaining a complete view of the disease. This will result in an informed approach to trial design and protocol development. It is extremely beneficial to obtain buy-in on the study design from KOLs, physicians, and site personnel to ensure that the study can be operationalized and executed efficiently. Equally important is ensuring regulatory authorities will support the study design, so it is imperative to have conversations early in the process. The CTI strategy encompasses all of these factors into our protocol development projects for rare disease indications.

Site & Investigator Identification

Once an executable protocol is complete, site and investigator identification is the next critical variable vital to the success of rare disease trials. It is important to look at the differences across geographies, whether there are differences in diagnostic equipment, opinions on treatment of the disease, current medications used to treat the disease, and regulatory stipulations for approval of the study. To answer these questions and help with site/investigator identification, a comprehensive feasibility questionnaire can be used. This feasibility can also help determine the number of patients each site expects to see with the disease, any site resources that could be helpful, and allows the PI and site personnel to weigh in on the protocol. It is also important to talk with sites and investigators about strategies related to site payments, publications, recruiting incentives, logistics, and any other issues early in the process to alleviate issues down the road. CTI's feasibility model includes direct peer-to-peer interaction from a CTI medical director to the investigator and the CTI study manager to the study coordinator on every rare disease program. Relying on electronic means of communication alone is insufficient to set the stage for a successful trial in complex and difficult disease areas.

Patient Population Identification

Patient identification, recruitment, and retention is one of the most challenging aspects of rare disease trials. It is important to utilize all available avenues of data to identify the patient population for a study in rare disease, including hospital databases, support/advocacy groups, published literature, and reaching out to physicians and KOLs in the disease area. Looking at the severity of the disease, life expectancy, prevalence in adult vs. pediatric, and examining current treatments are also important to patient identification. It is also important to work with sites to develop patient identification plans that

CTI Cares Spotlight

Cranio Care Bears is a charity that was begun by two mothers of boys with craniosynostosis. Its mission is to spread awareness, support, and compassion through loving care packages to families of children facing surgery for craniosynostosis. The care packages include items for the child and the family to relieve the stress accompanying this very serious surgery. Cranio Care Bears also offers one-on-one support before, during, and after surgery. The charity also strives to bring awareness of craniosynostosis to families and the medical community for early detection.

Nominated by: George Ranson

To learn more and donate

95% of CTI's Regulatory and Health Outcomes group has direct experience with rare disease protocol development and study design, and 100% of CTI's medical affairs team has direct experience with medical monitoring and protocol/study design in rare disease trials.

Recruitment & Retention

The impact of simplifying logistics for study participants is often an overlooked area in patient recruitment and retention. Patients may be all over the world, and many of them are children, which means logistics can be even more complicated. Special consideration should be given to travel arrangements for both the patient and family. Depending on their location, patients may need to be transported to another country or city to participate in a trial, and this may often mean uprooting whole families for a period of time. Trials may need to include a budget to support housing or other miscellaneous reimbursements for the patient and their family. The CTI team has worked collaboratively with our rare disease sponsors and individual sites to develop site plans that take patient/family logistics into consideration. These plans provide solutions for travel challenges and innovative mitigation strategies to minimize inconvenience to patients and families due to loss of work time, study visit schedules, as well as other factors which are often unique to each rare disease protocol.

Investigational Product Considerations

In addition to complicated patient logistics, there are usually equally complicated conditions around drug shipment, storage, and administration to consider as many investigational therapies for rare disease populations involve new and innovative products. These include cell and gene therapies in addition to first-in-class or first-in-population compounds. The use of any new and unique investigational product puts an emphasis on the development of procedures and training on those procedures to ensure appropriate shipment, storage, and administration. With limited patient numbers, there is no room for error placing critical importance on investigational product considerations. The CTI team has successfully managed global rare disease trials involving cell and gene therapies, in addition to therapies involving complex routes of administration and higher regulatory scrutiny due to their first-in-class and/or first-in-population nature.

More than 90% of CTI's study team members have direct experience on rare disease trials, including those working in study management, monitoring, data management, biostatistics, regulatory affairs, safety, and quality assurance.

CTI is a leader in rare disease research evident by our track record of success. CTI has made significant contributions to many approved products, and our regulatory team has been a part of multiple expedited approval pathways for rare disease products. We firmly believe that the success of our sponsors is our success and welcome each and every opportunity to push life-changing therapies forward in rare and under served populations.

For more information about Rare Disease Day 2017,
visit http://www.rarediseaseday.org/

RAREDISEASEDAY.ORG

"With research, possibilities are limitless."

New Additions & **Upcoming Meetings We** **Join our Team!**

Rashidat Alatishe joins as Clinical Research Assistant

Caro Barth joins as Senior Director, Business Development Europe

Candice Boudreaux joins as Human Resources Coordinator

Erica Christensen joins as Study Manager I

Kathy Dabbelt joins as Study Manager I

Ilse Gonella joins as Director, Business Development Europe

Amy Hoeper joins as Study Manager II

Chris Laber promoted to Associate Manager, Business Development Operations

Conner Maxwell promoted to Senior Business Development Associate

Ellen Maue promoted to Study Coordinator

Mike Montani promoted to Assistant Director, Business Development Operations

Emily Munafo promoted to Senior Human Resources Generalist

Beth Newell promoted to Study Manager II

David Sonny joins as Associate Manager, Business Development Operations

Ashley Stevens joins as Clinical Safety Scientist I

Rebecca Thompson joins as Study Coordinator

Robert Wessel joins as Director, Business Development

2017 Life Sciences PA Annual Dinner
Philadelphia, Pennsylvania
March 9, 2017

American Association for the Study of Liver Disease - Emerging Trends
Washington, DC
March 18-19, 2017

ARM Advanced Therapies Summit
Barcelona, Spain
March 22, 2017

B4B Neurology Conference
Frankfurt, Germany
March 29 - April 1, 2017

ARM Cell & Gene Investor Day
Boston, MA
April 27, 2017

To schedule a meeting with us at one of these, please click here

individuals to fill these positions:

Clinical Research Associate (US, UK, Germany, France, Spain, Australia, Brazil, Korea, Taiwan, Japan, Argentina)

CRA Manager (Ulm, Germany)

Clinical Safety Scientist (Cincinnati, OH; Raleigh, NC)

Clinical Systems Analyst (Cincinnati, OH)

Clinical Trial Assistant (Ulm, Germany)

Clinical Trial Assistant Manager (Ulm, Germany)

Director, Regulatory and Scientific Affairs (Cincinnati, OH; Raleigh, NC; Philadelphia, PA)

Operations Manager, Japan

QA Auditor (Cincinnati, OH)

Regulatory Specialist (Ulm, Germany)

Study Coordinator (Cincinnati, OH)

Study Manager (Cincinnati, OH; Raleigh, NC; Philadelphia, PA; San Francisco, CA)

Travel Coordinator (Cincinnati, OH)

Click here for more information and to apply!