CTI CLINICAL TRIAL & CONSULTING

Newsletter

Where Life-changing Therapies Turn First™

www.ctifacts.com

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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,

CTI Cares Spotlight

CTI joined the American Heart Association's fight against heart disease by holding our 10th annual Go RED Day on Friday, February 5th.

You can learn more about this national cause to increase awareness and help women reduce their risk of heart disease at www.GoRedForWomen.org

At CTI Clinical Trial and Consulting Services, we're committed to working on life-saving trials, such as those in many rare disease areas.

In the U.S., any disease affecting fewer than 200,000 people is considered rare, so to increase awareness for those with rare diseases around the world, we are challenging every CTI employee to participate in our 200,000 Step Challenge.

Employees around the world will track their steps from February 1 - February 29 (Rare Disease Day) in a virtual step challenge.

Funds raised through the CTI Step Challenge will be donated to the

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 may include patient support groups, using social media, as well as patient/physician referrals to figure out where the patient population exists.

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. Of the rare disease products approved in 2015, CTI made significant contributions to 4 approved products with several more anticipated to receive FDA and/or EMA approval in 2016 and 2017. 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:
www.ctifacts.com
513.598.9290

Upcoming Meeting Spotlight

Alliance for Regenerative Medicine
4th Annual Cell and Gene Investor Day
New York, NY
March 22, 2016

International Niemann-Pick Disease Alliance and the Tuberous Sclerosis Alliance, both chosen because of the relationship to recent rare disease projects the company has worked on.

For more information about Rare Disease Day 2016, visit http://www.rarediseaseday.org/

CTI is proud to sponsor and attend this ARM event!

Click here to schedule a meeting with us while we're at the Cell and Gene Investor Day!

Recent CTI Presentations and Publications

The Effect of the Choice of Contrast Media on Renal Failure Events in Inpatient Cardiovascular Procedures
Acute Kidney Injury (AKI) & CRRT 21st International Conference on Advances in Critical Care Nephrology San Diego CA
February 16-19, 2016

The Direct Healthcare Expenditures of Mitral Valve Disease: Evidence from United States National Survey Data
American Heart Association Quality of Care and Outcomes Research (AHA QCOR) Phoenix, AZ
February 28 - March 1, 2016

The Effect of the Choice of Contrast Media on Renal Failure Events in Inpatient Interventional & Diagnostic Cardiovascular Procedures
China Interventional Therapeutics (CIT) China National Convention Center
March 17, 2016

Contrast Media Selection in Inpatient Interventional Cardiovascular Procedures
China Interventional Therapeutics (CIT) China National Convention Center
March 17, 2016

New Additions & Promotions at CTI

Felicia Cochran, PhD joins as Associate Director, Regulatory and Scientific Affairs

Colleen Colson promoted to Associate Director, Global Quality Assurance

Yolanda Hill joins as Patient Recruiter

Matt Hodskins promoted to Associate Director, Project and Proposal Management

Beverly Martinez, PhD joins as Senior Manager, Health Outcomes Research

Upcoming Meetings We Will Be Attending

Alliance for Regenerative Medicine 4th Annual Cell and Gene Investor Day
New York, NY
March 22, 2016

World Orphan Drug Congress USA 2016
Washington, DC
April 20-22, 2016

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

Join our Team!! We're looking for individuals to fill these positions:

Associate Director, Health Outcomes Research (Cincinnati, OH; Philadelphia, PA; Raleigh, NC)

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

Clinical Research Coordinator (Cincinnati, OH)

Information Technology Support Specialist (Cincinnati, OH)

Jayne Minham, RN, BSN joins as Assistant Director, Clinical Trials

Scott Strassels, PharmD, PhD joins as Assistant Director, Health Outcomes Research

Renee Sylvester joins as Regulatory Specialist I

Medical Director (Cincinnati, OH)

Regulatory Specialist (Cincinnati, OH)

Safety Scientist (Raleigh, NC)

Study Coordinator (Cincinnati, OH)

Study Manager (Cincinnati, OH; Raleigh, NC; Philadelphia, PA; San Francisco, CA; Ulm, Germany; Paris, France; Madrid, Spain)

Click here for more information and to apply!