February Newsletter

Making Life-changing Impacts on Rare and Underserved Patient Populations

CTI is a front-runner in rare and orphan disease research with the majority of our active studies involving rare disease indications. 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. CTI has successfully navigated expedited regulatory pathways for rare diseases studies, recently including FDA Orphan Designation, FDA Fast Track Designation, FDA Rare Pediatric Disease Designation, FDA Regenerative Medicine Accelerated Therapy Designation, EMA Orphan Drug Designation, EMA Rare Disease Designation, and UK Innovative Medicine Designation, among others. 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 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 may include patient support groups, using social media, as well as patient/physician referrals to figure out where the patient population exists.

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. CTI's experience with patient recruiting and transportation logistics has included more than 30 countries around the world. 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.

Evident by our performance history and accomplishments, CTI continues to be a front-runner in rare and orphan disease research. We have an exceptional repeat business rate with our clients working in rare disease, which is a testament to our experience and success in the disease area. CTI has made substantial contributions to many approved products, and our regulatory team has been a part of multiple expedited approval pathways for rare disease products. The majority of our clinical team, including project management, clinical monitoring, data management, biostatistics, safety and pharmacovigilance, medical affairs, clinical systems, and regulatory affairs, have extensive experience in rare disease trials. We firmly believe that the success of our sponsors is our success and we welcome each and every opportunity to push life-changing therapies forward in rare and underserved patient populations.

Rare Disease Day 2018

CTI is thrilled to support and be a part of Rare Disease Day for the fifth consecutive year. This year's theme, **"With research, possibilities are limitless",** is particularly significant to the CTI team and the work we do each day to help life-changing therapies reach patients around the world. We are proud to be a part of rare disease research today and everyday.

Learn more about Rare Disease Day 2018

Rare Disease Day February 28, 2018

Additional Highlights

The CTI Way

There is a unique culture that exists at CTI, a method of approaching our work and interacting with our coworkers that we have come to refer to as "The CTI Way."

As we make our way towards our 20th year, each month we will be highlighting a different theme or aspect of "The CTI Way." The theme for the month of February is **"Grow."**

The employee of the month is awarded to the candidate who exhibits strength in CTI's core values, best exemplifies "The CTI Way" theme of the month, and consistently demonstrates outstanding performance.

Employee of the Month

Brianna Earle

Since joining the CTI team in May 2016, Brianna has always provided exceptional Safety management to her clients and has quickly grown into a leader within her department.

Her consistent high quality work, professionalism, and dedication to her clients has allowed CTI to grow additional business.

Brianna exemplifies the CTI Core Values specifically through the outstanding job she does every day, the safety scientist and leader she has become, and the commitment she displays to clients.

Congratulations, Brianna, on your well-deserved award!

Upcoming Meetings We'll be Attending

AKI-CRRT
San Diego, CA
March 6-9

ARM's Advanced Therapies Summit
Amsterdam, The Netherlands
March 14

EBMT Annual Meeting
Lisbon, Portugal
March 18-21

ASCPT Annual Meeting
Orlando, FL
March 21-24

eyeforpharma Philadelphia 2018
Philadelphia, PA
April 10-11

EASL
Paris, France
April 11-15

ARM's Cell & Gene Therapy Investor Day
New York, NY
April 17

DIA Europe
Basel, Switzerland
April 17-19

AMCP Managed Care & Specialty Pharmacy Annual Meeting

Boston, MA
April 23-26

International Translation and Regenerative Medicine Conference
Rome, Italy
April 25-27

The European Conference on Rare Diseases & Orphan Products (ECRD)
Vienna, Austria
May 10-12

World Advanced Therapies & Regenerative Medicine Congress
London, England
May 16-18

NASH Biomarkers Workshop
Washington, DC
May 18-19

ISPOR 23rd Annual International Meeting
Baltimore, MD
May 20-23

Outsourcing in Clinical Trials East Coast
Philadelphia, PA
May 22-23

ASCO
Chicago, IL
June 1-5

American Transplant Congress (ATC)
Seattle, WA
June 2-6

Digestive Disease Week (DDW)
Washington, DC
June 2-6

SLEEP 2018
Baltimore, MD
June 2-6

Bio International Convention
Boston, MA
June 4-7

36th Vicenza Course on AKI & CRRT
Vicenza, Italy
June 12-14

23rd Congress of EHA
Stockholm, Sweden
June 14-17

Join Our Team!

We are currently seeking qualified individuals to join our team!

Search Open Positions

CTI Cares

This month, the CTI family is raising money to support 'Project Alive'. The mission of Project Alive is to help find a cure for Hunter Syndrome (MPS II) through research and advocacy.

Learn more and donate

New Hires & Promotions

CTI is thrilled to welcome all of our new employees, and to congratulate our recently promoted employees!

View New Hires and Promotions

CTI in the News:

CTI Announces Promotions of Todd Bonta and Shawna Bredek

CTI Clinical Trial and Consulting Services (CTI), a multi-national, privately held, full-service contract research organization is pleased to announce the promotions of Todd Bonta and Shawna Bredek to Director, Clinical Data Management and to Senior Director, Clinical Trials respectively.

Read on »

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