CTI CLINICAL TRIAL & CONSULTING
Where Life-Changing Therapies Turn First™

April Newsletter

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Natural History Studies Are Vital to Clinical Trial Design

Contributed by Ryan Gifford - Senior Manager, Business Development and Client Management

Natural History Studies are an important tool that can reduce risk in trials when working in many disease areas, including rare disease indications. In many cases, little precedent exists for designing clinical trials in specific diseases indications, and there are inherent knowledge gaps related to design and appropriate clinical outcomes to measure. Through a Natural History Study, we can define the disease incidence, understand the variability of the disease, identify causes of morbidity and mortality, define a patient's lifespan, and perhaps most importantly, develop and validate tests along with outcome measures to ensure a clinial trial is adequently designed.

> *"For an intervention to receive FDA approval, research needs to demonstrate that the intervention has a clinically meaningful effect on patients through adequate and well-controlled studies. These studies must be based on a scientific foundation that includes knowledge of the disease's natural history."*
>
> Anne Praiser, M.D. Associate Director for Rare Diseases, Office of New Drugs, CDER, FDA – Workshop on Natural History Studies of Rare Diseases – NIH Campus, Bethesda, MD May 16-17, 2012 Workshop Summary

Success Stories From Natural History Studies

In some cases, patients in Natural History Studies can serve as historical controls, reducing not only the risk of the prospective trial, but also greatly reducing costs. The 2012 approval of Voraxaze®, a highly successful and life-saving drug that CTI worked on, is an example of the FDA appproving a drug based on historical control data.

Types of Natural History Studies

- Literature Reviews
- Retrospective Chart Reviews
- Prospective Cross-sectional Studies
- Prospective Longitudinal Studies

CTI's team of epidemiologists, statisticians, and physicians can help your team better understand the disease, and in parallel, develop and validate disease specific scoring or patient-reported measures as part of a Natural History Study for use in a prospective clinical trial. Depending on the types of questions you need answered, CTI can help design the appropriate type of Natural History Study to support your clinical development.

Benefits of Natural History Studies are Multi-factorial

Clinical Risk	Study Objectives	Valuation
Study design, statistical powering, site selection	*Determine Disease Incidence*	Market size
Endpoint considerations	*Understand Variability of the Disease*	Identification of undiagnosed or under diagnosed patients
Endpoint selection expected AE's	*Identify Causes of Morbidity and Mortality*	Impact of disease, Quality of life
Protocol design	*Understand Patients Lifespan*	Reimbursement considerations
Benchmark disease progression compared to treatment	*Define Disease Progression*	Reduce risk of AEs associated with investigational product
Endpoint selection		Proof of concept

CTI Cares Spotlight

National Multiple Sclerosis Society

Nominated by Jennifer Palmer, Clinical Research Coordinator II

Team Palmer's Passion participated in the MS Walk, Western & Southern Cincinnati 2014 event, that took place Saturday, April 26, 2014.

The National MS Society is a collection of passionate individuals who want to do something about MS now—to move together toward a world free of multiple sclerosis. MS stops people from moving. We exist to make sure it doesn't.

We help each person address the challenges of living with MS through our 50-state network of chapters. The Society helps people affected by MS by funding cutting-edge research, driving change through advocacy, facilitating professional education, and providing programs and services that help people with MS and their families move their lives forward.

Click here to learn more and donate

surrogate biomarker considerations	*Validate Outcome Measures*	definition, Reduction of study duration

For more information:

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Upcoming Meetings We'll be Attending:

Heart Rhythm Society Meeting
San Francisco, CA - May 7 -10

Israel Innovation Conference (MIXiii)
Tel Aviv, Israel - May 20 - 22

International Society Pharmacoeconomics and Outcomes Research 19th Annual Meeting
Montreal, QC - May 31 - June 4

Drug Information Association Annual Meeting 2014
San Diego, CA - June 15 -19

2014 BIO International Conference
San Diego, CA - June 23 -26

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

New Additions & Promotions at CTI

Brian Johnston promoted to Associate Director, Clinical Trials

Kelly Solinsky promoted to Senior Study Manager

Kirsten Cooney promoted to Study Manager

Tara Hutchins promoted to Clinical Data Manager

Chika Okere promoted to Assistant Project Manager

Christie Crosby promoted to Senior Study Coordinator

Andreas Lorenz, PhD joined as Director, Project Management Europe

Kwadwo Kwarteng joined as a Senior Biostatistician

Kristin Jones, PhD joined as a Senior Medical Writer

Laura Wright, PhD joined as a Senior Medical Writer

Sandra Comstock joined as a Study Manager

Ashley Perry joined as an Assistant Project Manager

Tyler Meer joined as a Research Associate

Kristen Robb joined as a Research Associate

Conner Maxwell joined as a Research Associate

Sabine Schoentaube joined as an Administrative Assistant - EU

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

Clinical Research Associate (US, Spain, UK, Germany, France, Belgium, Poland, Australia, Brazil)

Director, Health Outcomes Research (Cincinnati, OH)

Information Technology Support Specialist (Europe)

Quality Assurance Auditor (Cincinnati, OH)

Quality Assurance Auditor (Europe)

Regulatory Specialist (Cincinnati, OH)

Study Manager (Cincinnati, OH)

Patient Recruiter (Cincinnati, OH)

Click here for more information and to apply!