CTI CLINICAL TRIAL & CONSULTING

March Newsletter

Where Life-changing Therapies Turn First™

www.ctifacts.com

Volume 12, Issue 3

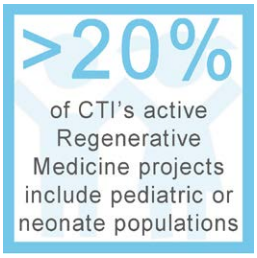
Overcoming Challenges in Regenerative Medicine

Regenerative medicine is an exciting and popular topic, engaging everyone from the biotechnology and pharmaceutical communities, to the everyday individual. The straightforward concept of using healthy cells or organs grown in the laboratory to repair or replace damaged, diseased or decaying organs has tremendous appeal. Similarly, gene therapy research is on the rise, as using a gene to treat or prevent a disease has sparked significant interest among both the pharmaceutical and biotechnology industries and patients alike. However, despite the significant increase in activity in the regenerative medicine arena over the last several years, the safe, effective, and efficient development of a unique product that is commercially viable and meets complex regulatory requirements remains a challenge.

CTI is a leader in regenerative medicine drug and device development. We understand the specific challenges these projects present, including regulatory hurdles, complex logistics around biological sample collection, shipment, and implantation, as well as complicated site training requirements based on unique cell and gene technologies. Our experience includes both autologous and allogeneic stem cells as well as other cell/gene interventions. We work with innovators, key opinion leaders, cutting edge biotechnology/pharmaceutical companies, and investigators on our ongoing programs, as well as participate in industry groups such as the Alliance for Regenerative Medicine (ARM).

Executing a successful program

A well-defined product or process and a clear developmental plan are an excellent start – however, successfully designing, enrolling, and completing the necessary clinical trials in the regenerative medicine field is difficult. Target patient populations can be anything from asymptomatic, early-stage disease to critically ill, late-stage patients. Regenerative medicine programs often include neonate or pediatric populations for optimal clinical benefit, which adds an additional challenge in the development and trial execution processes. This requires special attention to be given to entire family units in the design of recruitment and retention strategies, rather than patients alone. Well-trained, knowledgeable study managers and monitors are mandatory to succeed in cell and gene-based therapies. **Partnering with an organization, such as CTI, that has global expertise in clinical trial design and management experience in regenerative medicine can drastically improve chances of successful product development.**



Overcoming start-up challenges

Operational experience can often overcome the hurdles that are unique to regenerative medicine programs, such as an ultra-orphan disease trial that requires innovative ways to find subjects. These programs can require a wide geographic reach to obtain adequate patient numbers, and can also present the challenge of moving unique cell and gene-based therapies across international borders. Wide geographic distribution also introduces the challenge of working with multiple regulatory authorities, all with limited experience in cell-gene based therapies resulting in the potential for inconsistent regulatory requirements from country to country. **CTI has multiple operational experts with global regenerative medicine experience who can assist in proactively navigating the complex start-up processes required in a global execution situation.**

CTI Cares Spotlight

LEUKEMIA & LYMPHOMA SOCIETY®
fighting blood cancers

Leukemia & Lymphoma Society

The Leukemia & Lymphoma Society (LLS) is the world's largest voluntary (nonprofit) health organization dedicated to funding blood cancer research and providing education and patient services. Since 1949, LLS has been dedicated to curing leukemia, lymphoma and myeloma.

Click here for more information and to donate!

Obtaining regulatory approval

The ultimate commercial success of a safe, effective, and beneficial regenerative medicine product requires regulatory approval by the FDA and EMA. Expertise and experience in managing interactions with the FDA and EMA specific to regenerative medicine is crucial to obtaining that approval. Whether the goal is a BLA submission or a PMA/510(k) registration, each step of the approval process can be particularly challenging. Though currently limited, the growing regulatory experience with cell and gene-based products allows for more interactive dialogue to create novel solutions for unique programs in therapeutic areas where little or no precedent exists. However, optimally utilizing their expertise requires outside advice and experts in the arena who have experience with all phases and types of meetings. These experts must also have significant scientific understanding of the cell and gene therapy field and have specific experience in regulatory pathway negotiations. **A knowledgeable and experienced team of scientists, medical directors, and regulatory experts is critical to effectively and successfully address regulatory hurdles in the regenerative medicine arena. CTI has a team of these experts with years of regenerative medicine experience.**

CTI is a leader in regenerative medicine research

We are currently supporting 14 sponsors and 25 active projects across 20 countries. Our team is committed to the future of regenerative medicine, stemming from a desire to support innovative organizations and unique products with the potential to bring life-changing therapies to the patients that need them.

To learn more about these methods and how we may be of assistance, please contact us.

For more information:
www.ctifacts.com
513.598.9290

Recent CTI Publications

5 abstracts have been accepted by ISPOR and will be presented during the meeting in May

Upcoming Meeting Spotlight

American Transplant Congress Annual Meeting
Philadelphia, PA
May 2 - 6, 2015

Stop by and visit us at
Booth #1000 throughout the meeting!

To schedule a meeting with us while we're here, please click here.

Upcoming Meeting Spotlight

International Society for Pharmacoeconomics and

Tweet Button

Outcomes Research
20th Annual International Meeting
Philadelphia, PA
May 16 - 20, 2015

Stop by and visit us at Booth #711 throughout the meeting!

To schedule a meeting with us while we're here, please click here.

Upcoming Meetings We Will Be Attending

European Association for the Study of Liver (EASL) - International Liver Congress
Vienna, Austria - April 22 - 25

American Transplant Congress (ATC) - Annual Meeting
Philadelphia, PA - May 2 - 6

International Society for Pharmacoeconomics and Outcomes Research (ISPOR) - 20th Annual International Meeting
Philadelphia, PA - May 16 - 20

Future Leaders in the Biotech Industry
New York, NY - May 20

Drug Information Association (DIA) - 51st Annual Meeting
Washington DC - June 14 - 18

Bio International 2015 Convention
Philadelphia, PA - June 15 - 18

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

New Additions & Promotions at CTI

Dan Beach promoted to Senior Visualization Analyst

Michael Ryan promoted to Senior Manager, Data Analytics

Therese Swaisgood promoted to Senior Clinical Coding Specialist

Sandra Wöhr promoted to Clinical Systems Coordinator Europe

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

Clinical Research Associate (US, Germany, France, Australia, Brazil)

Director, Health Outcomes Research (Cincinnati, OH)

Manager, Proposal Development (Cincinnati, OH)

Project Accountant (Cincinnati, OH)

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

Click here for more information and to apply!