CTI
Clinical Trial and Consulting Services

# April Newsletter

Volume 11, Issue 4

**We are recruiting for CRAs in the US, UK, France, Belgium, Sweden, Germany, Poland, Italy and the Netherlands!**

**Upcoming Medical Meetings CTI will be Attending ...**

Stem Cell Commercialization and Partnering Conference
Boston, MA
April 29 – 30

Alliance for Regenerative Medicine Annual Dinner and Legislative Fly-In
Washington, DC
May 7 – 8

American Society of Gene and Cell Therapy
Salt Lake City, UT
May 15 – 18

American Society of Transplantation
Seattle, WA
May 18 – 22

## Role of Clinical Trials in Advancing Gene Therapy

Gene therapy has been discussed as a potential method to treat genetic disease since the advent of gene cloning in the early 1980's. The basic premise is that each gene codes for a protein that must be expressed in a specific time sequence and in certain cells for a human body to function normally. When a gene has a mutation or abnormal expression this may lead to one of the more than 1800 genetic diseases in humans.

Gene therapy has been progressing for a number of years with starts and stops due to various advances and concerns. One of the challenges with gene therapy is the use of various types of viral vectors, which act as transporters of the corrected gene's DNA into cells. There have been concerns that the viral vectors may recombine and reactivate an unwanted viral or immune activity in the recipient of the therapy. Another challenge is the possibility of insertional mutagenesis, when the corrected gene inserts into the human DNA in a place that either disrupts the function of another gene or enhances unwanted expression of a gene. The disruption or aberrant gene expression may result in the introduction of other diseases in the recipient. Since these situations have arisen over the years this has resulted in rigorous regulatory authority guidance and scrutiny for gene therapy trials. While somewhat daunting, both scientific and medicinal advances have allowed gene therapy trials to continue**. The November 2012 approval of Glybera® (alipogene tiparvovec) by the European Commission as a gene therapy to treat familial hyperchylomicronemia, a rare and inherited disease, marks the first gene therapy approval by a western regulatory authority.**

Currently there are a number of ongoing clinical trials exploring gene therapy as a curative therapy in somatic (non reproductive) cells in humans. In these trials there is concerted rigor in all aspects. There is regulatory guidance for the development of gene therapy products ranging from the preclinical aspects to the long-term safety considerations and follow-up needed on patients in gene therapy trials. Of special concern are the informed consent documents that need to clearly explain the potential risk, both short-term and long-term, to the patient in terms that are comprehensible to patients of young ages as well as their guardians.

**If you are interested in scheduling a meeting with CTI, please contact Nick Schatzman at 513-598-9290 or via email at nschatzman@ctifacts.com**

## Employee Update

Congratulations to the following CTI employee recently promoted:

**Allison Schroeder –** Associate Manager, Marketing and Corporate Communications

Please welcome the newest additions to CTI:

**Angela Bowens** – Clinical Safety Scientist

**Tom Winrod** – Associate Director, Clinical Trials

**Roy Wells** – Sr. Auditor, Quality Assurance

## Quick Links…

Our Website

Email

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Since many of the potential gene products involve a transfection of an individual's cells to essentially create a personalized gene therapy product, a number of logistical and Good Manufacturing Processes (GMP) concerns must be addressed. There must also be a defined chain of custody of a recipient's cells both before and after the genetic manipulation with appropriate analytic and GMP testing of the final cellular product. It is very important that only the recipient receive their personalized gene therapy product in order to avoid possible autoimmune rejection issues. All these logistical issues can be addressed through careful planning, documentation, release testing, and documented chain of custody of the product.

**CTI has a 14 year history of conducting complex clinical trials with complicated logistics related to transplantation studies. This experience and expertise is now being applied to the conduct of several ongoing gene therapy trials.** The results of these trials pave the path for the potential curative product for genetic diseases that currently have an unmet medical need.

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# About CTI

CTI Clinical Trial and Consulting Services is an innovative, international drug and device development organization that delivers a full spectrum of clinical trial and consulting services from bench to commercialization with a focus on immunology and a passion for helping life-changing therapies succeed in chronically and critically ill patient populations. CTI's focused therapeutic approach provides pharmaceutical, biotechnology and startup firms with clinical and disease area expertise from a unique mix of academic, medical and industry specialists; rich intellectual capital in transplantation, immunology, infectious diseases, hematology, cardiology, nephrology, hepatology, regenerative medicine and rare diseases; flexible study designs that accelerate development programs and deliver high approval ratings that are among the best in the industry; and exceptional global project management and gold standard safety and data management systems that strengthen their program's success potential. Established in 1999 and headquartered in Cincinnati, OH; CTI has offices in North America, Europe and South America.