CTI CLINICAL TRIAL & CONSULTING

Where Life-changing Therapies Turn First®

Newsletter

www.ctifacts.com

CTI Safety in Gene Therapy

Team and Technology Background

CTI Safety Team

CTI's safety team is led by our Director of Global Safety and Pharmacovigilance, Shelly Brewer. Shelly and her team have been a part of numerous CTI clinical trials in regenerative medicine, including multiple gene therapy studies in rare diseases.

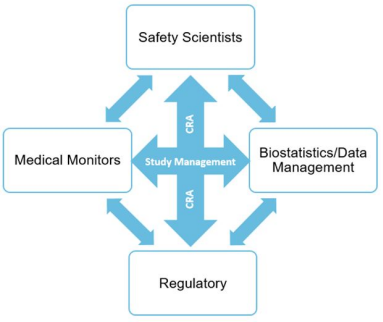
CTI's global safety and pharmacovigilance team includes experienced individuals with nursing, pharmacy, and other health professional backgrounds.

CTI Safety Technology

CTI offers:

- Fully validated safety database – Argus
- 24/7 SAE Hotline and eFax coverage
- Central CTI Safety email inbox
- Medwatch / CIOMS generation and submission
- Line listings and ad hoc report generation

Interactive Approach



Gene Therapy

CTI is a recognized industry leader in the regenerative medicine field. CTI's Global Safety and Pharmacovigilance team is well versed in gene therapy, having significant experience with trials involving both complex patient populations and unique gene therapy technologies.

Typically, regenerative medicine trials involve conditioning regimens, resulting in immunocompromised patients and a higher frequency of SAEs.

CTI Safety understands the unique demands of clinical trials involving rare/complex indications and novel therapies. Some of the rare/complex indications CTI has been involved with include, sickle cell disease, beta thalassemia, ovarian cancer, mesothelioma, lymphoma, adrenoleukodystrophy and mucopolysaccharidosis.

Individual gene therapy trials typically require separate Argus databases, however for long-term follow up requirements, a single follow up database can be utilized for events involving the same vector with different indications allowing report generation and analysis across indications involving a single vector. Reports can be run for an

CTI Cares Spotlight:

This month, CTI is taking the opportunity to care for one of our own.

CTI is committed to helping those in need, especially in communities and areas directly related to our employees, sponsors, and partners. We're focused on making life-changing therapies available for patients, but kindness, caring and generosity, can also be life-changing, and can help make this world a better place.

individual database, or across the enterprise and can consist of individual case reports or aggregate line listings.

We draw on our extensive clinical and drug development experience, working closely with clients, team members and sites to ensure thorough and accurate management of safety events. We provide 'turnkey' services across the drug development spectrum, including individual case management utilizing CTI's Argus Safety database, regulatory reporting (individual and aggregate), and Safety Surveillance to identify and assess emerging safety trends. We are currently responsible for safety activities on more than a dozen different gene therapy trials. Highlighted below are some of the gene therapy trials we are currently managing.

- Phase I/II Autologous T Lymphocytes Expressing Antibody Coupled T-cell Receptors
    - 4 sites
    - 23 patients
    - Primary endpoint – safety
- Phase I AAV vector gene transfer
    - 8 sites
    - 12 patients
    - Primary endpoint – safety and tolerability
- Phase I/II Autologous T-Cells Transduced with gamma retrovirus
    - 7 sites
        - 39 patients
    - Primary endpoint – safety and efficacy
- Phase I Autologous T cells transfected with mRNA
    - 2 sites
    - 30 patients – adult females
    - Primary endpoint – safety and efficacy
- Phase II/III Hematopoietic Stem Cells Transduced with Lentiviral Vector
    - Countries: US, Argentina, Australia, France, UK
    - 5 sites
    - 24 patients – boys age 17 and under
    - Primary endpoint – safety and efficacy
- Phase I/II Subretinal administration of DNA transduced vector Primary endpoint – safety and tolerability
    - 3 sites
    - 15 patients
- Phase I Transplantation of Autologous Stem Cells Transduced with Lentiviral Vector
    - 8 sites
    - 26 patients
    - Primary endpoint – safety

If you're interested in talking to CTI Global Safety about managing your next Regenerative Medicine trial call Shelly Brewer at (513) 598-9290 or email Shelly at sbrewer@ctifacts.com.

Posters and Presentation

DISEASE-RELATED COST BURDEN IN PATIENTS UNDERGOING SINUS SURGERY FOR CHRONIC RHINOSINUSITIS: A CLAIMS-BASED ANALYSIS
Hunter TD1, DeConde AS2, Maines RP3
1CTI Clinical Trial and Consulting Services, Inc., Covington, KY, USA, 2UC San Diego, San Diego, CA, USA, 3Yale School of Medicine, New Haven, CT, USA

ISO-OSMOLAR CONTRAST MEDIA AND ADVERSE RENAL AND CARDIAC EVENTS AFTER PERCUTANEOUS CARDIOVASCULAR INTERVENTION
McCullough PA1, David G2, Todoran TM3, Brilakis ES4, Ryan MP5, Gunnarsson C5
1Baylor Heart and Vascular Institute, Dallas, TX, USA, 2The Wharton School, University of Pennsylvania, Philadelphia, PA, USA, 3Medical University of South Carolina, Charleston, SC, USA, 4Minneapolis Heart Institute, Minneapolis, MN, USA, 5CTI Clinical Trial and Consulting Services, Covington, KY, USA

Upcoming Meeting Spotlight

CTI is a proud sponsor and will be attending this meeting November 9th.

New Additions & Promotions at CTI

Pavels Anetko, MD promoted to Assistant Director, Clinical Operations APAC

José-Luis Cascales promoted to Senior Clinical Project Manager

Upcoming Meetings We Will Be Attending

Advanced Therapies Investor Day
London, UK
November 9

Data, Evidence and Access Summit 2017
Philadelphia, PA

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

Clinical Quality Assurance Auditor (Greater Cincinnati, OH Area; Raleigh, NC)

David Flick promoted to Associate Manager, Information Technology

Elena Hazenbiller promoted to Regulatory Specialist I

Jaclyn Hemmerle promoted to Regulatory Specialist I

Chelsea Jones promoted to Study Manager I

Rosemary Lane joins as Clinical Project Manager, Europe

Molly McKean promoted to Senior Project and Account Manager

Jamina Rahmani joins as Clinical Trial Budget Manager, Europe

Tracy Reed-Kessler promoted to Study Manager II

Alexandra Stevenson moves to new role as Clinical Safery Associate

Kristina Wriston promoted to Manager, Business Development and Client Management

November 13-14

Partnerships in Clinical Trials
Amsterdam, Netherlands
November 28-29

American Society of Hematology Annual Meeting & Expo
Atlanta, GA
December 9-12

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

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

Clinical Research Coordinator (Greater Cincinnati, OH Area)

Clinical Safety Scientist (Greater Cincinnati, OH Area; Raleigh, NC)

Director, Clinical Trials (Greater Cincinnati, OH Area; Raleigh, NC Area)

IT Support Specialist (Greater Cincinnati, OH Area)

Project Manager (Greater Cincinnati, OH Area)

Quality Assurance Advisors (Ulm, Germany)

Research Scientist, Real World Evidence (Greater Cincinnati, OH Area)

Senior/Principal Research Scientist, Real World Evidence (Greater Cincinnati, OH Area)

Study Coordinator (Greater Cincinnati, OH Area)

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

Senior Medical Writer, Outcomes Research / Scientific Communications (Greater Cincinnati, OH Area; Raleigh, NC)

Click here for more information and to apply!