HESI CT-TRACS*: a Collaborative Effort to Address Collective Challenges & Needs.

*Cell Therapy-TRAcking, Circulation, & Safety (CT-TRACS) Technical Committee

William Shingleton, BSc, PhD
Alliances Manager, Cytiva
CT-TRACS Committee Co-Chair

PACT Web Seminars, September 3, 2020
HESI Cell Therapy-TRAcking, Circulation, & Safety (CT-TRACS) Technical Committee

What is HESI CT-TRACS and how can we contribute?
Health and Environmental Sciences Institute (HESI)

- Non-profit scientific organization based in Washington DC USA
- Address risk assessment and safety challenges.
- Operating Internationally.

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After 30 years, we KNOW the model works.

Bringing together **multidisciplinary teams** to solve the tough scientific challenges around **risk assessment and safety**.

- Facilitates collaboration across academia, government, industry, and NGO scientists.

Generating/making available/disseminating **sound, evidence-based science** for better, more informed decisions.

**Move Knowledge into Application**: creating and testing technology platforms and scientific frameworks that can be used to more effectively predict effects on humans or the environment.
CT-TRACS’ Vision

SECURING THE SAFETY OF CELL THERAPIES TO REALIZE THEIR POTENTIAL

MISSION:
To facilitate the translation of cell-based therapies to the clinic by driving the development of tools, methods and knowledge required to evaluate safety and fate of therapeutic cells.
**Universities/ Research Centers:**

- Imperial College London
- Ghent University
- Stanford University
- Cardiovascular Institute
- UCL
- Memorial Sloan Kettering Cancer Center
- Radboud University

**CT-TRACS Members**

HESI

*(2020 data)*

**CTPs developers, CROs, tool providers:**

- AstraZeneca
- Athersys Inc.
- Boehringer Ingelheim
- Bristol Myers Squibb
- Cell Dynamics International
- CSL Behring
- Cytiva
- Janssen
- Merck
- Novartis
- Sanofi
- Takeda
- VisiCELL Medical

**Government & Regulatory bodies:**

- FDA
- MHRA
- Medicines Evaluation Board
- NIH
- National Institute for Public Health and the Environment
- RIVM
- Ministry of Health, Welfare and Sport

**NGOs / Consortia:**

- CATAPULT
- eatris
- FIRM
- PACT

*Product Assistance for Cellular Therapies*

- National Heart Lung and Blood Program

>70 Participants

>30 Organizations
Rationale for creating CT-TRACS

Novel Therapies, new safety assessment needs:

• “Living drugs” - Need to evaluate “cell fate” after administration of cell therapies, i.e., the localization, persistence, migration, distribution and/or eventual phenotypic changes of cells, in the patient.
• Conventional PD/PK methodologies not always transferrable to CGT.
• Regulatory landscape not clearly defined, yet.
• Need for stakeholders’ interaction.

➢ Issues that are best resolved through international multi-sector interaction, pooling resources and knowledge for a common goal.
Safety Testing: Time & experience for different drug types

Safety, DMPK, ADME

Small Molecule

Large Molecule

Cell & Gene Therapy

Idea
Molecule synthesis
Pre-clinical
Manufacture
Clinical Trials

Safety, DMPK, ADME

Stem cell transplants, Allogeneic?

Autologous e.g. CAR-T?

History of drug development (years)
Rationale for creating CT-TRACS

Dawn of a New Era
Strategies to help translate cell therapies into the clinical setting.

**Step 1:** Identifying knowledge gaps

CT-TRACS’ stakeholder survey 2017: Assessing the needs for cell-based therapies translation

Cross-sector discussions in CT-TRACS monthly teleconferences

**Step 2** – Establish focused groups to address issues identified

PoA: Localization, persistence, survival, proliferation.

Biodistribution: Types of effects, ability to detect, ability to assess fate and distribution.

**Step 3** – Strategies to fill gaps

- Knowledge sharing across sectors and organizations
- Review paper (Helfer et al. In submission)
- Public resources, making data accessible: Cell Tracking Database
- Communication & International Outreach: HESI workshops, sessions, presentations at conferences.

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CT-TRACS:

CT-TRACS established, scoping and membership developed.

Year-2: needs and gaps identification; outreach.

Fall 2015 to 2016

2017

2018

2019

Jan 2018: Approved by HESI BoT for elevation to HESI “Technical Committee”.

2019:
1st larger outputs of the committee; Launch of new projects

2020:
Publications Collaborative, multi-site studies

Options for Imaging cellular therapeutics in vivo: a multi-stakeholder perspective

Running title: imaging cellular therapeutics in vivo


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A channel to communicate challenges and needs
Mechanisms for practical solutions

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<td>Scientific staff</td>
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*Projects address a need or issue relevant to a broad cross-section of the scientific community (academia, industry, government) and have public health significance.*
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