



ISCT 2017: HESI CT-TRACS Session Description

“Identifying and Optimizing Emerging Technologies to Evaluate Cell Therapy Safety, Mode of Action and Efficacy”

Saturday May 6th, 2017. 15:00 – 16:30, London, UK.

Session Chair: Dr. William Shingleton (GE Healthcare, CT-TRACS co-chair)

1. **Introduction:** HESI CT-TRACS structure, mission, goals and collaborations (5 min) - *Chair*
2. **Current developments:** (presenters from CT-TRACS and two guest speakers, 10 min each)
 - a. **Tracking cells after administration/biodistribution:**
 - i. Imaging modalities and probes (Dr. *Brooke Helfer, Celsense, CT-TRACS sub-group co-leader*)
 - ii. Imaging Cell Delivery Near The Bed: Are We There Yet? (case-studies) – *Prof. Jeff Bulte, Director of Cellular Imaging, The Johns Hopkins Institute for Cell Engineering.*
 - iii. Preclinical and clinical biodistribution study in cell-based therapeutic product development (*Dr. Nobuhiro Umeda, Astellas*)
 - b. **Tumorigenicity assessment:**
 - i. Tumorigenicity Assessment of Human Cell-Based Therapeutic Products (*Dr. Yoji Sato, Head of the Division of Cell Based Therapeutic Products, NIHS Japan.*)
 - ii. Tumorigenicity Evaluations of AST-OPC1: Oligodendrocyte Progenitor Cells for the Treatment of Spinal Cord Injury (case-study) – *Dr. Jane Lebkowski, President of R&D and Chief Scientific Officer, Asterias*
3. **Panel Discussion** – panelists will include the presenters from part 2. Session Chair will facilitate the discussion. Focus on gaps and needs in an interactive discussion with the audience. Seeking feedback from therapy developers and regulators (30 minutes).

Learning Objectives and Speakers Biographies

Session Chair: Dr. William Shingleton (GE Healthcare, CT-TRACS co-chair)

Dr. William Shingleton:

Biological Scientist, with a research background in inflammation, biology of ageing and connective tissues. Applying this research in the fields of auto-immune and degenerative diseases. Experience gained in academia at the Universities of Cambridge and Newcastle-upon-Tyne, combined with over 13 years of industrial research with Unilever R&D and GE Healthcare. Recent roles have been focussed on supporting the Cell Therapy Industry through development of manufacturing tools and technology and the application of *in-vivo* imaging to enable safety, efficacy and MoA studies.



Talk 1: Tracking cells after administration: imaging modalities and probes.

Dr. Brooke Helfer, Celsense, CT-TRACS sub-group co-leader

Learning objectives:

- To learn about the different imaging modalities for monitoring cellular therapeutics.
- To learn about developing and presently available clinically applicable cell tracking technologies.
- To learn about how imaging can aid in the translation of cellular therapeutics

Dr. Brooke Helfer is the Director of Research and Development at Celsense, Inc. Celsense is a Pittsburgh Pennsylvania based company that specializes in translating cellular imaging for human health by providing tools to non-invasively visualize, characterize and measure biological processes in living systems. Dr. Helfer's primary research interests relate to translational cancer immunotherapy, promoting the development of cellular therapeutics, and the advancement of future scientists. Dr. Helfer completed her Ph.D. in Cancer Cell Biology in the Mary Babb Randolph Cancer Center at the West Virginia University Health Science Center, and her B.A. in Biology and German from Washington and Jefferson College in Washington, PA.

Talk 2: Imaging Cell Delivery Near The Bed: Are We There Yet?

Prof. Jeff Bulte, Director of Cellular Imaging, The Johns Hopkins Institute for Cell Engineering.

Learning objectives:

- To learn about the currently available, clinically applicable cell tracking/imaging techniques. There are two ways of making cells detectable: labeling cells with exogenous nanoparticles or endogenous cellular expression of reporter genes.
- To learn how cell tracking can aid in optimizing the practice of clinical cell therapy. This includes conducting image-guided cell injections, visualization of cell homing and migration, and assessment of cell survival.
- To learn about a first-in-man clinical trial on tracking cells using fluorine-based magnetic resonance imaging and the key steps that were involved for obtaining clinical approval.

Jeff W.M. Bulte, Ph.D., is a Professor of Radiology, Oncology, Biomedical Engineering, and Chemical & Biomolecular Engineering at the Johns Hopkins University School of Medicine. He serves as the Director of Cellular Imaging in the JHU Institute for Cell Engineering. He received his PhD degree summa cum laude From the University of Groningen, The Netherlands in 1991. Dr. Bulte spent 10 years at the National Institutes of Health in the Laboratory of Diagnostic Radiology Research, first as a postdoctoral fellow and then as a Staff Scientist. He was recruited to Johns Hopkins University as an Assistant Professor in 2001, became an Associate Professor in 2002, and a Full Professor in 2006. He is a Fellow and Gold Medal awardee of the ISMRM and a Distinguished Investigator of the Academy of Radiology Research. He has published over 235 peer-reviewed publications and 40 book chapters, with a current h-index of 78.



Talk 3: Preclinical and clinical biodistribution study in cell-based therapeutic product development.

Dr. Nobuhiro Umeda, Astellas Pharma Inc.

Learning objectives:

- To review what cell tracking/imaging studies were performed in cell therapy product development both in clinical and preclinical phases.
- To learn how the biodistribution assessment studies benefit cell therapy product development by reviewing method, tool and design of these studies.
- To find out strategy of the best application of biodistribution assessment for the benefit of clinical translation of cell therapies.

Nobuhiro Umeda, Ph.D., is a Senior researcher, Bioimaging department, Translational Science Research Labs., Astellas Pharma Inc. He received his PhD degree from the University of Tokyo, Japan in 2011. Dr. Umeda spent 3 years each in Pharmacokinetics/ Pharmacodynamics Research and Translational Science Research in Astellas.

Talk 4: Tumorigenicity Assessment of Human Cell-Based Therapeutic Products.

Dr. Yoji Sato, Head of the Division of Cell Based Therapeutic Products, NIHS Japan.

Learning objectives:

- Establish a basic strategy for the control of tumorigenicity in the development and manufacturing of cell-based therapeutic products.
- Describe the performance and limitations of testing methods associated with tumorigenicity evaluation of cell-based therapeutic products.
- Discuss challenges and possibilities associated with the establishment of international standardization/harmonization of tumorigenicity assessment of cell-based therapeutic products.

Dr. Yoji Sato is Head of Division of Cell-Based Therapeutic Products, National Institute of Health Sciences (Tokyo). He is also an Adjunct Professor of Graduate School of Pharmaceutical Sciences, Nagoya City University, a Guest Professor of Graduate School of Pharmaceutical Sciences, Osaka University, and an Adjunct Professor of Graduate School of Pharmaceutical Sciences, Kyushu University. He received his Ph.D. in Pharmaceutical Science from the University of Tokyo in 1995. While a post-doctoral fellow at the University of Cincinnati College of Medicine, he succeeded in establishing a variety of transgenic animal models to elucidate mechanisms of cardiac excitation-contraction coupling. Dr. Sato's current research area is in the field of regulatory science for the quality and safety of cell-based therapeutic products. He is also serving as a member of a Technical Committee of the Ministry of Health Labour and Welfare, and as a board member of the Japanese Society for Regenerative Medicine.



Talk 5: Tumorigenicity Evaluations of AST-OPC1: Oligodendrocyte Progenitor Cells for the Treatment of Spinal Cord Injury (case-study).

Dr. Jane Lebkowski, President of R&D and Chief Scientific Officer, Asterias Biotherapeutics Inc.

Learning objectives:

- Current tumorigenicity testing typically requires long-term evaluation of cell therapy products in immunocompromised animals.
- Careful consideration of the dose, location, delivery method and intended duration of cell activity must be made when designing tumorigenicity assessments of cell-based therapies.
- Clinical trials should be designed with appropriate periodic imaging techniques to monitor for possible tumorigenicity and mitigate adverse effects.

Jane Lebkowski has been actively involved in the development of cell and gene therapies since 1986 and is currently President of R&D and Chief Scientific Officer at Asterias Biotherapeutics Inc, where she is responsible for all R&D of Asterias' products. From 1998 to 2012, Dr. Lebkowski was Senior Vice President of Regenerative Medicine and Chief Scientific Officer at Geron Corporation. Dr. Lebkowski led Geron's human embryonic stem cell program, being responsible for all research, preclinical development, product development, manufacturing, and clinical development activities. Prior to Geron, Dr. Lebkowski was Vice President of Research and Development at Applied Immune Sciences. Following the acquisition of Applied Immune Sciences by Rhone Poulenc Rorer (currently Sanofi), Dr. Lebkowski remained as Vice President of Discovery Research. Dr. Lebkowski received her Ph.D. in Biochemistry from Princeton University, and completed a postdoctoral fellowship at the Department of Genetics, Stanford University.

Last 30 minutes: Session Chair and all speakers invited on stage for a Panel Discussion

This program was proposed as part of a collaborative effort of the HESI (Health and Environmental Sciences Institute) Cell Therapy - TRACKing, Circulation, & Safety committee ([CT-TRACS](#)). CT-TRACS is a public-private collaboration of scientists with a shared interest in addressing current challenges and identifying best practices to ensure a safe translation of cell-based therapies into the clinic. [HESI](#) is a non-profit, scientific organization based in Washington, DC but operating globally. HESI's scientific programs develop science involving academic, government, and industry partners who combine resources and expertise to address contemporary issues in human and environmental health and safety.

For additional information, please contact Dr. Lucilia Mouriès, HESI Scientific Program Manager:
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For more information about HESI visit www.hesiglobal.org.