PROGRAM

Thursday, September 12, 2019

16:00 – 19:00  REGISTRATION  Lakeside Commons (Level 1)
16:00 – 19:00  SPEAKER SERVICES  Meeting Room N

Friday, September 13, 2019

07:00 – 19:00  REGISTRATION  Lakeside Commons (Level 1)
07:00 – 19:00  SPEAKER SERVICES  Meeting Room N

07:30 – 08:30  MORNING SESSION 1 – MSC THERAPY; RATIONALISM VS. EMPIRICISM; HOW MUCH MUST WE KNOW TO TEST THERAPIES IN PATIENTS; A LIVE DEBATE  Ballroom A
Moderator: Allan Dietz
Speakers:
Jacques Galipeau – MSC – Is in vivo Persistence the Key to Potency? A Rational Approach for Pragmatic Trials
William Faubion – Application of Cell Therapies to Human Disease

07:30 – 08:30  QUALITY & OPERATIONS TRACK SESSION 1 – MEDIA FILLS  Ballroom B
Chair: TBC
Speakers:
Adrian Gee – Validation of Media Fills
Aisha Khan – Media Fills: Aseptic Process Simulation for Cell Therapy Products Manufacturing

08:45 – 10:15  PLENARY SESSION 1 – MESENCHYMAL AND PROGENITOR STEM CELLS: RESULTS FROM THE CLINIC AND THE FUTURE OF LIVING DRUGS  Plenary Hall
Chair: Allan Dietz
Speakers:
Allan Dietz – Development of Autologous Adipose Derived Mesenchymal Stromal Cell Therapy; Selected Clinical Results and Lessons Learned from Treating More than 200 Patients Under IND
Gil Van Bokkelen – Addressing Major Areas of Unmet Medical Need with “Off The Shelf” Cell Therapy
Etienne Sokal – Human Allogeneic Liver Progenitor Cells (HALPC) (HepasStem®) Clinical Development for Acute on Chronic Liver Failure and NASH

10:15 – 10:45  COFFEE BREAK
**Friday, September 13, 2019**

<table>
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<th>Time</th>
<th>Session</th>
<th>Location</th>
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<tbody>
<tr>
<td>10:45 – 12:15</td>
<td>WORKSHOP 1 – NAVRMA JOINT SESSION – VETERINARY MEDICINE IN DEVELOPING CELL-BASED THERAPIES AND BIOENGINEERING APPROACH FOR HUMAN CLINICAL USE</td>
<td>Plenary Hall</td>
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<td>Co-Chairs: Daniel Weiss and Tracey Webb</td>
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<td>Speakers:</td>
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<td></td>
<td>Andrew Hoffman – Companion Animal Models Scaffold to Regenerative Medicine Therapeutics in Humans</td>
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<td>Kyla Ortved – Cell &amp; Gene Therapies for Joint Disease and Cartilage Repair Using the Horse as a Model for Human Disease</td>
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<td>Aimee Colbath – Equine Models of Joint Disease for the Investigation of Cell-Based Therapies</td>
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<td>Dori Borjesson – How Naturally Occurring Disease Models in Dogs and Cats Inform Human Clinical Trials</td>
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<td>10:45 – 12:15</td>
<td>QUALITY &amp; OPERATIONS TRACK SESSION 2 – MANAGING THE MUSHROOMING GROWTH IN CELL AND GENE THERAPY</td>
<td>Ballroom B</td>
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<td>Chair: Lynn O'Donnell</td>
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<td>12:15 – 13:45</td>
<td>LUNCH</td>
<td>Exhibition Hall</td>
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<td>12:45 – 15:15</td>
<td>PLENARY SESSION 2 – IPSC AND PLURIPOTENT STEM CELLS – TRUE REPLACEMENT CELL THERAPIES</td>
<td>Plenary Hall</td>
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<td>Chair: Robert Deans</td>
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<td>Speakers:</td>
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<td></td>
<td>Dan Kaufman – Engineered iPSC-derived Lymphocytes For Improved Cancer Therapy</td>
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<td>Stephanie Protze – Cell Replacement Therapy for the Conduction System: Biological Pacemakers</td>
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<td>Robert Deans – Cell Therapy for the Brain: Neuronal and Glial Based Therapeutics</td>
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<td>15:15 – 15:30</td>
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**Friday, September 13, 2019**

**WORKSHOP 2 – PTF SESSION – REGULATORY ASPECTS OF UNPROVEN CELL THERAPIES IN NORTH AMERICA**

*Plenary Hall*

Chair: Laertis Ikonomou  
Speakers:  
- Peter Marks – FDA’s Approach to Investigational Cell Therapies  
- Lynne Boxer – FDA Regulation of Animal Cells, Tissues, and Cell- or Tissue-Based Products  
- Ajay Kuriyan – The Pitfalls of Unregulated Cell Therapy in Ophthalmology  
- Kirstin Matthews – Right to Try Laws and Expanding Access to Experimental Stem Cell Interventions

**WORKSHOP 3 – GENE REPLACEMENT THERAPY USING ADULT STEM CELLS**

*Ballroom A*

Chair: Mitchell Cairo  
Speakers:  
- Mitchell Cairo – Hematopoietic Stem Cell Transplantation and Gene Therapy for Hemoglobinopathies  
- Jeffrey Medin – Gene Therapy for Fabry Disease Using Autologous CD34+ Hematopoietic Cells  
- Paul Orchard – Hematopoietic Stem Cell Transplantation and Gene Therapy for Cerebral Adrenal Leukodystrophy (CALD)

**QUALITY & OPERATIONS TRACK SESSION 3 – STABILITY**

*Ballroom B*

Chair: Karl Stasko  
Speakers:  
- Karl Stasko – A Road Best Traveled: AABB–ISCT Joint Working Group Stability Committee Update  
- Jackie Damen – The CFU Assay: A Biological Tool to Determine Functional Viability, Potency and Stability of CTPs  
- Rosemarie Bell – Developing and Executing a Stability Program

**BREAKING BOTTLENECKS – THE TRAINING, DEVELOPMENT, AND STAFFING CHALLENGE**

*Plenary Hall*

Co-Hosted by: ISCT ESP Committee and CellCAN.  
Speakers:  
- Emily Hopewell, PhD, Indiana University School of Medicine, United States  
- Craig Hasilo, MSc, PhD(C), CellCAN, Canada  
See p. 35 for detailed description.

**Saturday, September 14, 2019**

**REGISTRATION**  
*Lakeside Commons (Level 1)*

**SPEAKER SERVICES**  
*Meeting Room N*
### Saturday, September 14, 2019

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<tr>
<th>Time</th>
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<tr>
<td>07:30 – 08:30</td>
<td>MORNING SESSION 2 – MEET THE EXPERTS: CELLULAR THERAPY QUALITY AND OPERATIONS</td>
<td>Plenary Hall</td>
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|               | Chair: Lizette Caballero  
Panelists: Paul W. Eldridge, Olive J. Sturtevant |                   |
|               | MORNING SESSION 3 – MEET THE EXPERTS: CAR T AND OTHER CELL “VEHICLES”    | Ballroom A        |
|               | Chair: Sarah Nikiforow  
Speaker: Bruce Levine |                   |
| 08:45 – 10:15 | QUALITY & OPERATIONS TRACK SESSION 4 – CURRENT CLINICAL DEVELOPMENT REGULATORY GUIDANCE – CONTEXT AND IMPLICATIONS | Ballroom B        |
|               | Chair: Kurt Gunter  
Speakers:  
Peiman Hematti – Regulatory Considerations for More Than Minimally Manipulated Cell-Based Therapies: A Clinician Perspective  
Cynthia Molina – Clinical Regulatory Guidance for Genetically-Modified Cell Therapies: An Industry Perspective |                   |
| 10:15 – 10:45 | PLENARY SESSION 3 – REDIRECTING IMMUNE CELLS – T AND NK CELLS ASIDE FROM CANONICAL CARS | Plenary Hall      |
|               | Chair: Sarah Nikiforow  
Speakers:  
Denis-Claude Roy – T Cell Approaches to Fight Infections and Leukemia without Causing Graft-Versus-Host Disease  
Sumithira Vasu – Off-The-Shelf Natural Killer Cell-Based Therapies for Malignancies  
Lisa Butterfield – Genetic Engineering Cells for Solid Tumors |                   |
| 10:45 – 12:15 | WORKSHOP 4 – OPTIMIZING CLINICAL TRIALS IN CELLULAR THERAPY | Plenary Hall      |
|               | Chair: Denis-Claude Roy  
Speakers:  
Lauralyn McIntyre – Mesenchymal Stromal Cells for Sepsis: the Potential and the Challenges  
Sarah Nikiforow – Balancing Flexibility With Standardization to Bring Cell Therapy Trials to Fruition  
Steven Devine – Role of Outcomes Registries in Cellular Therapy |                   |
| 12:00 – 13:00 | COFFEE BREAK | Exhibition Hall |
| 10:45 – 12:15 | ADVANCED PRACTICE PROFESSIONALS (APP) TRACK SESSION 1 – MANAGEMENT OF CRS TOXICITY OR OTHER REACTIONS RELATED TO CAR THERAPY | Ballroom A        |
|               | Chair: Valkal Bhatt  
Speakers:  
Christina Bachmeier, Rebecca Gonzalez, Marian Dam – Pumping the Breaks On CAR-T Cell Toxicities |                   |
| 12:00 – 13:00 | ORAL ABSTRACTS SESSION 2 | Ballroom B        |
|               | Chair: Jacques Galipeau  
PRESENTATIONS:  
Rita Barcia – Phase I/II Exploratory Biomarker Interim Analysis of Extracorporeal Mesenchymal Stromal Cell Therapy (SBI-101) Therapy in Subjects With Acute Kidney Injury (Abstract 7)  
Raghavan Chinnadurai – IFN-Activated Allogeneic Mesenchymal Stromal Cells Mitigate Lethal Hematopoietic Radiation Injury in Association With Sparing of Marrow Endogenous CD11b+CD11c+ Myeloid Compartment. (Abstract 8)  
Michael Schrod – Apoptotic Mesenchymal Stem Cells Polarize Macrophage Subsets to an Immunosuppressive State (Abstract 9)  
James Ankrum – 3D Aggregation of Human MSCs Alters their Immunomodulatory Potency (Abstract 10)  
Ashley Krull – MSc Gene Expression Changes in Platelet Lysate-Free Media and Cerebrospinal Fluid Provide Direct Applicability to Human Clinical Trial (Abstract 11)  
Lorena Braid – Secreted Responses of Umbilical Cord Mesenchymal Cells to Full Thickness Swine Burns are Refined by the Zone of Injury (Abstract 12) |                   |
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<td>LUNCH</td>
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<td>12:30 – 13:30</td>
<td>CORPORATE TUTORIAL&lt;br&gt;<strong>Hosted by Roche CustomBiotech</strong>&lt;br&gt;<strong>Speaker:</strong> Sowmya Viswanathan, PhD, University Health Network Cell Therapy Program, Canada</td>
<td>Ballroom A</td>
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<td>13:45 – 15:15</td>
<td>WORKSHOP 5 – ENGINEERING TO CONTROL CELL ENVIRONMENT DURING PROCESSING&lt;br&gt;<strong>Chair:</strong> Erika Trauzzi&lt;br&gt;<strong>Speakers:</strong>&lt;br&gt;James Lim – Designing Cell Therapies to Work Effectively in Immunosuppressive Tumor Microenvironment&lt;br&gt;Peter Beemiller – Synthetic Activation for Efficient Expansion of Antigen Specific T-Cells&lt;br&gt;Angelo Cardoso – Engineering Hematopoietic Stem Cells – An Academic Perspective</td>
<td>Plenary Hall</td>
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<td>13:45 – 15:15</td>
<td>ADVANCED PRACTICE PROFESSIONALS (APP) TRACK SESSION 2 – RESPONSIBILITIES OF THE INVESTIGATOR AND THE LAB IN THE INVESTIGATION, COMMUNICATION AND REPORTING OF ADVERSE EVENTS&lt;br&gt;<strong>Chair:</strong> Valkal Bhatt&lt;br&gt;<strong>Speakers:</strong>&lt;br&gt;Julia Thebiay – Responding to Drug Related Serious Adverse Events; the Manufacturer’s Obligation to the Sponsor and the PI&lt;br&gt;Ashwani Khurana – Proper Reporting of Drug Related Serious Adverse Events; Regulatory Obligations of the PI and Sponsor</td>
<td>Ballroom A</td>
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<td>15:15 – 15:45</td>
<td>COFFEE BREAK</td>
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Saturday, September 14, 2019

WORKSHOP 6 – CAR-T RACING: AMATEURS & ROOKIES LOOKING TO CONTRIBUTE
Plenary Hall
Chair: Patrick Hanley
Speakers:
- Lizette Caballero – Trial and Tribulations: Lessons Learned when Preparing for an Industry-Sponsored Clinical Study
- Paul Eldridge – DIY – CAR-T Cells: Flipping Your Cell Therapy Lab
- Hema Dave – Drive the Change: Bringing CAR-T Therapies to Your Institution

WORKSHOP 7 – REGULATORY UPDATES – WHAT YOU NEED TO KNOW WHEN FILING NOVEL IND’S
Ballroom A
Chair: Olive Sturtevant
Speakers:
- Jessica Chery – Regulatory Framework for Gene Therapies Incorporating Human Genome Editing – A CBER Perspective
- Amanda Mack – Considerations When Pursuing an iPSC-Based Clinical Product
- Whitney Gladney – Synthesizing and Editing a CRISPR IND for Submission and Acceptance

QUALITY & OPERATIONS TRACK SESSION 6 – STRATEGIES FOR INSTITUTING MATERIALS MANAGEMENT PROGRAM FOR CELL AND GENE THERAPY PRODUCTS
Ballroom B
Chair: Aisha Khan
Speakers:
- Robert Lindblad – Potential Impact of 21CFR Regulations on the Control of Components Used in Gene Transfer or Genomic Editing
- Yong Fan – Raw Material Qualification-Regulatory Perspective
- Lynn O’Donnell – Where the Rubber Hits the Road – Challenges, Work-Arounds, Compromises and Solutions for Materials Management

17:15 – 18:15
POSTER SESSION
Exhibition Hall

ISCT MEMBERS AND ESP NETWORKING RECEPTION
Madison Club (off-site)
Celebrating 17 Years of Service – John Barrett, MD, Senior Editor of Cytotherapy 2001-2018 and ISCT Mentor
Hosted by the ISCT North America Region
By invitation only

Sunday, September 15, 2019

07:00 – 12:00
REGISTRATION
Lakeside Commons (Level 1)

07:00 – 12:00
SPEAKER SERVICES
Meeting Room N

07:00 – 08:30
*Morning Session 4 – Meet the Experts: Virus-Specific T Cells
Ballroom A
Chair: Lynn O’Donnell
Speakers:
- Catherine Bollard – Virus Specific T-Cell Therapies – Broadening Applicability Beyond CMV and EBV
- John Barrett – Polyomaviruses
- Michael Grimley – Third Party Virus Specific T-Cells (VSTs): Cincinnati Experience

07:30 – 08:30
QUALITY & OPERATIONS TRACK SESSION 7 – SYNCHRONIZING CMC PREPARATION FOR COMMERCIAL APPROVAL WITH REGULATORY ACCELERATORS
Ballroom B
Chair: Yong Fan
Speakers:
- Mohammad Heidaran – Chemistry Manufacturing Control Readiness for Expedited Programs
- Marianna Sabatino – The CMC Pressure in Developing Fast Track Product
### Sunday, September 15, 2019

**PLENARY SESSION 4 – EMERGING ENGINEERING STRATEGIES IN REGENERATIVE MEDICINE**

*Chair: William Murphy*

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| 08:45-10:15 | Speakers:  
  - William Murphy – Engineering Autologous Therapies for Regenerative Medicine  
  - Timothy Kamp – Harnessing hPSC-derived Cardiac Progenitor Cells and Cardiac Fibroblasts for Myocardial Repair  
  - Mervin Yoder – Identifying Endothelial Stem Cells for Vascular Regeneration |

**COFFEE BREAK**

10:15 – 10:45

**WORKSHOP 8 – CAR-T RACING: THE PRO CIRCUIT + FUTURE PROSPECTS**

*Chair: Bruce Levine*

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| 10:45-12:15 | Speakers:  
  - Rebecca Gardner – Advancing CAR-T Cell Product Development for B-ALL to Overcome Resistance  
  - Krishanu Saha – New Frontiers in Gene Modification and Genetic Engineering of CAR-T Cells  
  - Saar Gill – CAR-T Cells for Myeloid Malignancies |

**ORAL ABSTRACTS SESSION 3**

*Chair: Karen Nichols*

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| 10:45 – 12:15 | Presentations:  
  - Daniel Tremmel – Reconstructing the In Vivo Niche for Islet Culture and Transplantation (Abstract 13)  
  - Sarah Rempel – Human Stem Cell-Derived Photoreceptors Generate Axons Through Non-Cell Autonomous Pulling (Abstract 14)  
  - Christopher Lazarski – Designing a High Throughput Multi-Parameter Assay for Process Development of Antigen Specific T-Cell Therapy Products (Abstract 15)  
  - Kevin Bosse – FDA-Regulated Research: Cellular Therapy Considerations (Abstract 16)  
  - Allan Dietz – The Mayo Clinic Cell Therapy Fellowship: Training the Next Generation of Cell Therapy Leaders (Abstract 17)  
  - Timothy Catlett – Abnormal axon guidance of human neurons in a model of Tuberous Sclerosis Complex is due to mTOR-independent defects in RhoA signaling. (Abstract 18) |

**QUALITY & OPERATIONS TRACK SESSION 8 – REAL WORLD EVIDENCE AND APPLICATIONS IN PRODUCT DEVELOPMENT**

*Chair: Jiwen Zheng*

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| 10:45 – 12:15 | Speakers:  
  - Adnan Jaigirdar – Real World Evidence: A Regulatory Perspective  
  - Leanne Larson – Trends and Challenges in Real-world Evidence Generation |