Welcome to Fabulous Las Vegas!

On behalf of everyone at ISCT and members of the entire cell therapy community, it is a true pleasure to welcome you to the 21st Annual Meeting of the International Society for Cellular Therapy in the fabulous city of Las Vegas.

It is generally said that “what happens in Vegas stays in Vegas...” well, we hope that this isn’t so because we have planned a conference with talks that are happening in Vegas but, I am sure, will be taken home and shared globally.

Las Vegas is famous for its mega casino-hotels and their entertainment. In these few days, we are delighted to be able to bring hundreds of delegates together from all over the world and, this time, to entertain them with cell-based translational research topics and applications embedded throughout an outstanding program.

We will see old and new friends, exchange ideas and learn about new concepts and technologies. This open and informal sharing of experiences, successes and also difficulties is the beauty of ISCT meetings. This resides within a collection of the most updated cell therapy experiences that are moving out from the laboratory environment to reach patients and their unmet needs. This is why we are here every year, to improve the performance of cellular therapies and to make them not only available, but safe. And we do see improvements every year!

ISCT follows these changes by bringing together different professionals in cellular research and translation: technologists, academics, manufacturing experts, clinicians, regulators, industry members and laboratory specialists. This balanced combination between academia and industry is truly unique and it is generating results that are presented here. They sit together, synchronize their efforts and share their achievements to move forward once again.

Our Meeting co-Chairs—Paul Eldridge, Jacques Galipeau and Hans Klingemann—with the precious support of the Organizing Committee, have put together plenary sessions, workshops and technical sessions on the most advanced concepts around cellular therapies.

Additionally, a workshop-enriched pre-conference day is planned for delegates on Global Regulatory Perspectives, Flow Cytometry and, in collaboration with other organizations, on Cord Blood and laboratory accreditation processes.

During the main meeting, eyes will be pointed at hundreds of slides presenting novel insights on new mesenchymal progenitors ontogeny for innovative tissue regeneration approaches and explaining how cell-based immunotherapy and tissue engineering may become game changers in medical care.

The more we move forward in this field, the more we have the opportunity to invite speakers to present advanced phase clinical trials developed within enabling policy and practice frameworks. Information on how to empower not-for-profit organization contributions to advanced cell therapy and, ultimately, to cell and tissue products commercialization are generously shared by the Speakers. Tissue specific regeneration workshops and technical sessions will bring fresh insights on lung, cardiovascular, neurological repair approaches, GVHD and CD34 cell processing.

A long-lasting and unique ISCT tradition, you will find a variety of interesting sessions in our Quality and Operations and Strategies for Commercialization Tracks. Do you want to build a cGMP facility? Do you have questions on ancillary reagents or potency assays? Are you burning to understand how the reimbursement of cell therapy products is being implemented? Ask them! You will be surprised how many answers you may have out of these Tracks. Lastly, the ISCT Annual General Meeting (AGM) will take a gamble and feature an innovative new area of cellular therapy research in space and its applications on earth.

This year we give a special welcome to our youngest investigators that can find new roles in a growing Society, thanks to the newly founded Early Stage Professionals Group. In the meantime, they will surely present data during oral and poster sessions to stimulate conversational exchanges that will be beneficial to the field and contribute to making them the ISCT leaders of the future.

Our industry participants are also very welcome partners. Particularly, this year I want to give a special thanks to our Exhibitors for their valuable support and I urge all of you to visit the exhibit hall. In addition, we cannot forget to offer a particular thanks to our industry partners and our generous sponsors.

In closing, I would like to thank Paul, Jacques, and Hans together with the entire Organizing Committee and ISCT Head Office for their incredible work and vision in preparing this Annual Meeting in Las Vegas.

In thanking all the delegates for their active participation, I will give you a suggestion: when leaving Las Vegas please consider the back of the iconic sign which reads: Drive Carefully and Come Back Soon so we can Connect, Communicate, and Translate!

Massimo Dominici, MD
ISCT President 2014-2016
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ISCT 2015: Coming of Age

On behalf of the entire ISCT 2015 Organizing Committee, we would like to extend to you a warm welcome to Las Vegas for the 21st Annual Meeting of the International Society for Cellular Therapy (ISCT). The program agenda over the next four days will be as dynamic and engaging as our host city. Cutting edge science, updates on advances in clinical applications of cellular therapy, and interactive networking opportunities will provide an exciting opportunity for attendees at all levels. The interaction of people is what makes ISCT such a strong and vibrant society. Connect, Communicate, Translate. These are the keywords for ISCT and its members.

Las Vegas is an astonishing place. Thomas Taj Ainlay describes it as “At night, it is a brilliant cluster of jewels of all shapes, sizes, and colors, glowing in the middle of a vast, black velvet canopy. By day, it is also an amazing sight, almost like a mirage. There is nothing but desert and rugged mountains all around, and then--in the middle of it all--one of the largest and fastest growing cities in the United States ... truly an enigma.” The energy and excitement that are hallmarks of Las Vegas reflect the enthusiasm of ISCT members for the fast growing field of cellular therapy. The pairing of ISCT and Las Vegas is very à propos as we see cellular therapy ideas mature into mainstream clinical therapies. Truly, cellular therapy and ISCT are coming of age together at this 21st meeting.

It has been said that everything and anything you want to do, you can do in Las Vegas. The same can be seen in the conference agenda. A diverse offering of six Plenary Sessions with topics including MSC biology, cellular immunotherapy, advanced phase clinical trial updates, enabling practices for commercialization, tissue engineering and the role of not-for-profit organizations in advancing cell therapies. Speakers represent all world regions making the conference a truly global event. Technical sessions and Workshops on multiple tracks provide platforms for the three pillars of ISCT: Academia, Regulatory and Quality/Operations, and Commercialization. Over 40 sessions will provide information on a broad range of topics from technical aspects of cell product manufacturing to regulatory and policy discussions to delivery of therapy to strategies for reimbursement. Basically, anything you want, you can do and a little bit more that you may not have realized. A new area of growth for ISCT is the inclusion of dedicated sessions for Advanced Practice Professionals. This reflects the strong growth of the clinical delivery of cell therapies seen worldwide. In addition to the session speakers, Oral Abstract and Poster presentations will provide a stimulating opportunity to interact with fellow attendees.
In addition to the conference program, there is an extensive set of pre-conference workshops on Wednesday, May 27th. These include workshops on Global Regulatory Perspectives, Flow Cytometry and FACT Training, as well as an inaugural dedicated Cord Blood workshop in partnership with the newly formed Cord Blood Association.

We are exceedingly grateful for the support of sponsors for the 21st Annual Meeting and encourage attendees to avail themselves of the opportunities to visit exhibit booths and attend corporate symposia and tutorials. These are always informative and a highly useful tool to make connections and learn about the latest technological advances.

We wish to thank all the speakers who have generously agreed to share their experiences at the conference. Thanks is also due to everyone who graciously helped in the organization of the conference. We have found that ISCT members are among the most helpful people in the world with unflagging enthusiasm, commitment and ability. This made creating this amazing program a joyful task.

We hope you have a highly informative and entertaining time with us here in Las Vegas, and thank you for joining us for this special event. ISCT Las Vegas – The Sky Is The Limit!

Jacques Galipeau, MD FRCP(C)
Emory University
Atlanta, USA

Paul Eldridge, PhD
University of North Carolina at Chapel Hill
Chapel Hill, USA

Hans Klingemann, MD, PhD
Conkwest Inc.
Cambridge, USA

www.isct2015.com
General Conference Information

REGISTRATION

<table>
<thead>
<tr>
<th>Date</th>
<th>Time</th>
<th>Location</th>
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<tr>
<td>Tuesday May 26</td>
<td>1400 – 1900</td>
<td>Promenade Foyer</td>
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<tr>
<td>Wednesday May 27</td>
<td>0700 – 1900</td>
<td>Promenade Foyer</td>
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<tr>
<td>Thursday May 28</td>
<td>0700 – 1700</td>
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<td>Friday May 29</td>
<td>0730 – 1700</td>
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<td>Saturday May 30</td>
<td>0730 – 1500</td>
<td>Promenade Foyer</td>
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INCLUDED IN YOUR ANNUAL MEETING REGISTRATION FEE:

- Access to the Welcome Address and Exhibit Open House and Welcome Reception
- Access to all scientific and educational sessions excluding the following pre-conference events:
  - Global Regulatory Perspectives (GRP) Workshop
  - Flow Cytometry Workshop
  - Cord Blood Workshop
  - FACT Cellular Therapy Inspection and Accreditation Workshop
- Lunch, all coffee breaks and refreshments served from May 28 – 30
- Academic Program
- Corporate Guide
- 2015 Abstract Supplement of Cytotherapy, the official journal of ISCT
- Access to the Conference Networking and Partnering App powered by JUJAMA
- Delegate Bag
- Access to presentations online (post-event)

WIFI

Available throughout the ISCT Annual Meeting Space

Network Name: ISCT2015
Password: PCT-2015

EXHIBIT-ONLY ATTENDEES RECEIVE:

- Access to the Exhibit Hall including the Welcome Address and Exhibit Open House and Welcome Reception on May 27
- Conference meals and refreshments served in the exhibit hall
- Corporate Conference Guide

SOCIAL EVENTS

WEDNESDAY MAY 27TH
WELCOME ADDRESS AND RECEPTION

ADMISSION:
Free for all conference delegates

WHERE & WHEN:
1900 – 1930: Welcome Address in the Roman Ballroom
1930 – 2130: Exhibit Open House and Welcome Reception (Exhibit Hall – Palace Ballroom)

Wine and hors d’oeuvres will be served

FRIDAY MAY 29TH
GALA EVENT

See Registration Desk for Details and Availability.

ADMISSION:
- $125 (ISCT Members)
- $95 (ISCT Member Technologists, APPs*, Trainees, Students, and Emerging Economies),
- $150 USD (Non-Members)

Purchase at Registration Desk

*APPs are Advanced Practice Professionals (advanced practice nurses/nurse practitioners, physician assistants, pharmacists)

WHERE & WHEN:
1900 – Midnight: Caesars Palace Roman Ballroom
Continuing Medical Education

CME

TARGET AUDIENCE

This activity has been designed to meet the educational needs of physicians and researchers in clinical practice focusing on malignancy/hematopoietic disease, autoimmune disorders, wound healing, cardiology, diabetes, neurology and primary immune deficiencies.

EDUCATIONAL OBJECTIVES

After completing this activity, the participant should be better able to:

• Describe the translational aspects of and issues involved with, all types of cell and tissue-based research
• Demonstrate cross-disciplinary participation from scientists, clinicians, laboratory personnel, regulatory professionals, and others from both academia and industry
• Identify the scientific, clinical, laboratory and regulatory issues related to each type of cell-based research/therapy
• Translate information and experience from more mature cellular research areas such as hematopoietic stem cell transplantation to other emerging areas including nonhematopoietic areas such as mesenchymal stem cells, islet cells, Esc's, etc.
• Initiate the transfer of information and experience from senior practitioners to young scientists in the field

FACULTY

Malcolm Brenner, Texas Children’s Cancer and Hematology Centers
Paul Carpenter, Fred Hutchinson Cancer Research Center/Seattle Cancer Care Alliance
Richard Childs, National Heart, Lung, and Blood Institute
Utkan Demirci, Stanford University
Allan Dietz, Mayo Clinic
Massimo Dominici, University of Modena
Tobi Fisher, UT MD Anderson Cancer Center
Kaj Fried, Karolinska Institute
Sarah Gilpin, Massachusetts General Hospital
Jeffrey Gimble, Tulane University
Bambi Grilley, Baylor College of Medicine
Alison Gulbis, MD Anderson Cancer Center
Helen Heslop, Baylor College of Medicine
Scott Hollister, University of Michigan
Christian Jorgensen, IRMB/INSERM

Kristen Kindsvogel, Fred Hutchinson Cancer Research Center/Seattle Cancer Care Alliance
Mauro Krampera, University of Verona
Joanne Kurtzberg, Duke University Medical Center
Ivan Martin, University Hospital Basel
Michael May, Centre for Commercialization of Regenerative Medicine
Simón Méndez-Ferrer, Spanish National Center for Cardiovascular Research (CNIC)
C. Randal Mills, California Institute for Regenerative Medicine
Katy Rezvani, UT MD Anderson Cancer Center
Jeff Ross, Miromatrix Medical Inc.
Luc Sensebé, Etablissement Français Du Sang EFS
Keith Thompson, Cell Therapy Catapult
Richard Vile, Mayo Clinic
Daniel J. Weiss, University of Vermont College of Medicine
Jennifer Wilson, UCSF School of Medicine

PHYSICIAN CONTINUING MEDICAL EDUCATION

Accreditation Statement

This activity has been planned and implemented in accordance with the accreditation requirements and policies of the Accreditation Council for Continuing Medical Education (ACCME) through the joint providership of Postgraduate Institute for Medicine and the International Society for Cellular Therapy. The Postgraduate Institute for Medicine is accredited by the ACCME to provide continuing medical education for physicians.

Credit Designation

The Postgraduate Institute for Medicine designates this live activity for a maximum of 10.25 AMA PRA Category 1 Credit(s)™. Physicians should claim only the credit commensurate with the extent of their participation in the activity.
PHARMACIST CONTINUING EDUCATION

Accreditation Statement
Postgraduate Institute for Medicine is accredited by the Accreditation Council for Pharmacy Education as a provider of continuing pharmacy education.

Credit Designation
Postgraduate Institute for Medicine designates this continuing education activity for 4.25 contact hour(s) (0.425 CEUs) of the Accreditation Council for Pharmacy Education.
(Specific Universal Activity Numbers – to be listed on certificate)

Type of Activity
Knowledge

DISCLOSURE OF CONFLICTS OF INTEREST
Postgraduate Institute for Medicine (PIM) requires instructors, planners, managers and other individuals who are in a position to control the content of this activity to disclose any real or apparent conflict of interest (COI) they may have as related to the content of this activity. All identified COI are thoroughly vetted and resolved according to PIM policy. PIM is committed to providing its learners with high quality CME activities and related materials that promote improvements or quality in healthcare and not a specific proprietary business interest of a commercial interest.

The faculty reported the following financial relationships or relationships they or their spouse/life partner have with commercial interests related to the content of this continuing education activity:

<table>
<thead>
<tr>
<th>Name of Faculty or Presenter</th>
<th>Reported Financial Relationship</th>
<th>Name of Faculty or Presenter</th>
<th>Reported Financial Relationship</th>
</tr>
</thead>
<tbody>
<tr>
<td>Malcolm Brenner</td>
<td>Royalty; Bellicum PLC, Advisory board; Conkwest, Bluebird Bio, Cell Medica, FFCanvac. Stocks, Bluebird Bio, FFCanvac, Viracyte</td>
<td>Alison Gulbis</td>
<td>Has no real or apparent conflicts of interest to report</td>
</tr>
<tr>
<td>Paul Carpenter</td>
<td>Speakers Bureaus; Therakos. Contracted Research; Novartis</td>
<td>Helen Heslop</td>
<td>Royalty; Cell Medica-EBV specific T cells. Contracted Research; Celgene. Stocks; Viracyte.</td>
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<tr>
<td>Richard Childs</td>
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<td>Scott Hollister</td>
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<td>Utkan Demirci</td>
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<td>Christian Jorgensen</td>
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<tr>
<td>Allan Dietz</td>
<td></td>
<td>Kristen Kindsvogel</td>
<td></td>
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<tr>
<td>Massimo Dominici</td>
<td></td>
<td>Mauro Kramperera</td>
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<tr>
<td>Tobi Fisher</td>
<td>Has no real or apparent conflicts of interest to report</td>
<td>Joanne Kurtzberg</td>
<td>Has no real or apparent conflicts of interest to report</td>
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<tr>
<td>Kaj Fried</td>
<td>Has no real or apparent conflicts of interest to report</td>
<td>Ivan Martin</td>
<td>Has no real or apparent conflicts of interest to report</td>
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<tr>
<td>Sarah Gilpin</td>
<td></td>
<td>Michael May</td>
<td>Has no real or apparent conflicts of interest to report</td>
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<tr>
<td>Jeffrey Gimble</td>
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<td>Simón Méndez-Ferrer</td>
<td>Has no real or apparent conflicts of interest to report</td>
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<tr>
<td>Bambi Grilley</td>
<td>Has no real or apparent conflicts of interest to report</td>
<td>C. Randal Mills</td>
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</tr>
<tr>
<td>Jeff Ross</td>
<td>Ownership Interest; Miromatrix Medical Inc.</td>
<td>Katy Rezvani</td>
<td>Has no real or apparent conflicts of interest to report</td>
</tr>
<tr>
<td>Luc Sensebé</td>
<td></td>
<td>Daniel J. Weiss</td>
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</table>
The planners and managers reported the following financial relationships or relationships they or their spouse/life partner have with commercial interests related to the content of this continuing education activity:

The following PIM planners and managers, Trace Hutchison, PharmD, Samantha Mattucci, PharmD, CHCP, Judi Smelker-Mitchek, RN, BSN and Jan Schultz, RN, MSN, CHCP, hereby state that they or their spouse/life partner do not have any financial relationships or relationships to products or devices with any commercial interest related to the content of this activity of any amount during the past 12 months.

DISCLOSURE OF UNLABELED USE

This educational activity may contain discussion of published and/or investigational uses of agents that are not indicated by the FDA. The planners of this activity do not recommend the use of any agent outside of the labeled indications.

The opinions expressed in the educational activity are those of the faculty and do not necessarily represent the views of the planners. Please refer to the official prescribing information for each product for discussion of approved indications, contraindications, and warnings.

CMLE

This continuing medical laboratory education activity is recognized by the American Society for Clinical Pathology as meeting the criteria for 20 hours of CMLE credit. ASCP CMLE credit hours are acceptable to meet the continuing education requirement for the ASCP Board of Registry Certification Maintenance Program. California CMLE credits are also available for 20 hours.

METHOD OF PARTICIPATION AND REQUEST FOR CREDIT

There are no fees for participating and receiving CMLE credit for this activity. CMLE credits are offered for all Plenary Sessions, Technical Sessions, Workshops, Quality and Operations Track Sessions, Strategies for Commercialization Track Sessions, and Oral Presentation Sessions from May 28-30, 2015.

In order to receive credit for this activity, participants must complete the online evaluations for the sessions they attend. Please visit www.isct2015.com to complete the evaluation form. Online evaluation must be completed by June 19, 2015.

CMLE certificates will be sent by email within 4-6 weeks of the program end date.
Program at a Glance

Wednesday May 27th

1630 – 1830 Corporate Symposium hosted by Miltenyi Biotec
Florentine Ballroom III/IV

1900 – 1930 Welcome Address
Roman Ballroom

1930 – 2130 Exhibit Open House and Welcome Reception
Exhibit Hall – Palace Ballroom

Thursday May 28th

0800 – 0900 Concurrent Sessions (Strategies for Commercialization Track 1 starts at 07:30)
Florentine Ballroom/Pompeian Ballroom

0900 – 1045 Opening Remarks and Presidential Plenary Session on Dissecting MSC Heterogeneity From Unexpected Ontogeny to Specialized Functions
Roman Ballroom

1115 – 1230 Concurrent Sessions
Florentine Ballroom/Pompeian Ballroom/Capri Room

1245 – 1345 Corporate Tutorials (see Corporate Guide for details)
Florentine Ballroom/Pompeian Ballroom

1400 – 1530 Plenary Session 2 on Cellular Immunotherapy
Pompeian Ballroom

1600 – 1730 Concurrent Sessions
Florentine Ballroom/Pompeian Ballroom/Capri Room

1730 – 1900 Poster Session 1
Palace Ballroom Foyer

1730 – 1900 Global to Local Reception
Exhibit Hall – Palace Ballroom

1830 – 2030 ISCT Early Stage Professionals Reception
Location TBA

Friday May 29th

0800 – 0900 Concurrent Sessions
Florentine Ballroom/Pompeian Ballroom

0915 – 1045 Plenary Session 3 on Advanced Phase Clinical Trial Updates in Cell Based Therapies
Roman Ballroom

1115 – 1230 Concurrent Sessions
Roman/Florentine/Pompeian Ballroom

1230 – 1345 Presidential Task Force on the Use of Unproven Cellular Therapies Round Table
Roman Ballroom

1245 – 1345 Corporate Tutorials (see Corporate Guide for details)
Florentine Ballroom/Pompeian Ballroom

1400 – 1530 Plenary Session 4 on Game Changers: Policy and Practice Changes Enabling the Commercialization of Cell and Tissue Products
Pompeian Ballroom

1600 – 1730 Concurrent Sessions
Florentine Ballroom/Pompeian Ballroom

1730 – 1900 Poster Session 2
Palace Ballroom Foyer

1900 – midnight Gala Event • Tickets required
Roman Ballroom

Saturday May 30th

0800 – 0900 Concurrent Sessions
Florentine Ballroom/Pompeian Ballroom

0915 – 1045 Plenary 5 Session on Tissue Engineering
Roman Ballroom

1115 – 1215 Concurrent Sessions
Pompeian Ballroom

1230 – 1345 Annual General Meeting and Feature Presentation by NASA
Florentine Ballroom/Pompeian Ballroom

1345 – 1515 Concurrent Sessions
Florentine Ballroom/Pompeian Ballroom

1530 – 1535 Best Oral and Best Poster Awards Presentation
Roman Ballroom

1535 – 1700 Plenary 6 Session on Not-for-Profit Organizations and Contributions to Advancing Cell Therapies
Florentine Ballroom
Organizing Committee

MEETING CO-CHAIRS

Jacques Galipeau, MD, FRCP(C)  
Emory University  
Atlanta, GA, USA

Paul Eldridge, PhD  
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Karl Stasko, BSc, MPH  
Co-Chair, Quality and Operations Track  
Dana Farber Cancer Institute, Boston, MA, USA

Daniel J. Weiss, MD, PhD  
University of Vermont, Burlington, VT, USA

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Dana Farber Cancer Institute, Boston, MA, USA

MEMBERS

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ICTC, Lima, Peru

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TUMCells TU Munich, Munich, Germany

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Reliance Life Sciences Pvt Ltd., Mumbai, India

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Aby J. Mathew, PhD  
BioLife Solutions, Bothell, WA, USA

MEMBERS

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Pfizer Regenerative Medicine, Cambridge, MA, USA

William Milligan  
Steminent Biotherapeutics Inc., Vancouver, BC, Canada

Steve Oh, PhD  
Bioprocessing Technology Institute  
Singapore, Singapore

Claudia Zylberberg, PhD  
Akrorn Biotech, Boca Raton, FL, USA

Ohad Karniel, MBA, PhD  
Pluristem Therapeutics Inc., Haifa, Israel

Knut Niss, PhD  
Novartis, Morris Plains, NJ, USA

Antonio Lee, PhD  
Medi-Post, Seoul, South Korea
Invited Chairs and Speakers

Eytan Abraham, PhD, Lonza – United States
Julie Allickson, PhD, Wake Forest Institute for Regenerative Medicine – United States
Eduardo Almeida, PhD, NASA Ames Research Center – United States
Vicki Antonenas, MSc, Westmead Hospital – Australia
Sue Armitage, MD Anderson Cord Blood Bank – United States
Shirley Bartido, PhD, Charité – Universitätsmedizin Berlin – Germany
John Barrett, MD, National Institute of Health – United States
Steven Bauer, PhD, Food and Drug Administration – United States
Rosemarie Bell, B.App.Sc, MASM, QIMR Berghofer Medical Research Institute – Australia
René Benjamin, PhD, H.C. Wainwright & Co. – United States
Chotima Bötcher, PhD, Charité-Universitätsmedizin Berlin – Germany
Harvey Brandwein, PhD, Pall Corporation – United States
Christopher Bravery, PhD, Consulting on Advanced Biologicals Ltd. – United Kingdom
Malcolm Brenner, MD, PhD, Texas Children’s Cancer and Hematology Centers – United States
Thomas Brieva, PhD, Celgene Cellular Therapeutics – United States
Lizette Caballero, BSc, MT, University of California San Francisco – United States
Sarah Callens, Cell Therapy Catapult – United Kingdom
Andrew Campbell, Thermo Fisher Scientific, Inc. – United States
Paul Carpenter, MD, Fred Hutchinson Cancer Research Center – United States
Richard Childs, MD, National Heart, Lung, and Blood Institute – United States
Debra Christianson, Center for International Blood and Marrow Transplant Research – United States
Dominic Clarke, PhD, Charter Medical – United States
Jeffrey Cohen, MD, Cleveland Clinic – United States
Nick Crabb, PhD, National Institute for Health and Care Excellence – United Kingdom
Lynn Csontos, STEMCELL Technologies, Inc. – Canada
Emily Culme–Seymour, PhD, London Regenerative Medicine Network – United Kingdom
Heather Daley, Dana Farber Cancer Institute – United States
Moya Daniels, MSc, Fate Therapeutics, Inc. – United States
Anthony Davies, PhD, Dark Horse Consulting – United States
Francesco Dazzi, MD, PhD, Kings College – United Kingdom
Robert Deans, PhD, Athersys, Inc. – United States
Utkan Demirci, PhD, Harvard University – United States
Allan Dietz, PhD, Mayo Clinic – United States
Massimo Dominici, MD, University Hospital of Modena and Reggio Emilia – Italy
Dawn Driscoll, PhD, DCI Biotech, Inc. – United States
Suzanne Dworsky, MD Anderson Cancer Center – United States
Shannon Eaker, PhD, GE Healthcare – United States
Paul Eldridge, PhD, University of North Carolina – United States
Stephanie Farnia, MPH, National Marrow Donor Program – United States
David Fiorentini, MSc, Biological Industries – Israel
Tobi Fisher, MPAS, PA–C, University of Texas, MD Anderson Cancer Center – United States
Karen Foster, ViaCord – United States
Kaj Fried, DDS, PhD, Karolinska Institute – Sweden
Jacques Galipeau, MD, FRCP(C), Emory University - United States
Adrian Gee, PhD, Baylor College of Medicine – United States
Saar Gill, MD, PhD, University of Pennsylvania – United States
Sarah Gilpin, PhD, Massachusetts General Hospital – United States
Jeffrey Gimble, MD, PhD, Tulane University – United States
Deborah Griffin, MSc, ASQ CPGP, Moffitt Cancer Center – United States
Bambi Grilley, RPh, RAC, CIP, CCRC, CCRP, Baylor College of Medicine – United States
Alison Gulbis, PharmD, MD Anderson Cancer Center – United States
Patrick Hanley, PhD, Children’s National Medical Center – United States
Helen Heslop, MD, Baylor College of Medicine – United States
Scott Hollister, PhD, University of Michigan – United States
Emily Hopewell, PhD, MT, Moffitt Cancer Center – United States
Pamela Jacobson, University of Utah – United States
Christian Jorgensen MD, PhD, IRMB/INSERM – France
Ohad Karnieli, PhD, Pluristem Therapeutics Inc. – Israel
Carolyn Keeever–Taylor, PhD, Medical College of Wisconsin – United States
Panos Kefalas, Cell Therapy Catapult – United Kingdom
Aisha Khan, ABD, MSc, MBA, Interdisciplinary Stem Cell Institute, University of Miami – United States
Kristen Kindsvogel, ARNP, Fred Hutchinson Cancer Research Center/Seattle Cancer Care Alliance – United States
Jason Kolbert, MBA, Maxim Group – United States
Mauro Krampera, MD, University of Verona – Italy
Joanne Kurtzberg, MD, Duke University Medical Center – United States
Deborah Lamontagne, BSc, Florida Hospital Cancer Institute – United States
Natalia Lapteva, PhD, Baylor College of Medicine – United States
Katarina Le Blanc, MD, PhD, Karolinska University Hospital – Sweden
Thomas Leemhuis, PhD, University of Cincinnati – United States
Ann LeFever, PhD, Northwestern Memorial Hospital – United States
Aaron Levine, PhD, MPhil, Georgia Institute of Technology – United States
Bruce Levine, PhD, University of Pennsylvania – United States
Zihai Li, MD, PhD, Medical University of South Carolina – United States
Bangon (Day) Longsomboon, MSc, Interdisciplinary Stem Cell Institute, University of Miami, United States
Mark Lowdell, PhD, FRCPath, FSB, Univeristy College of London – United Kingdom
Geoff MacKay, Proteus Venture Partners – United States
Ivan Martin, PhD, University Hospital Basel – Switzerland
Jerry Martin, Pall Corporation – United States
Aby J. Mathew, PhD, BioLife Solutions, Inc. – United States
Michael May, PhD, Centre for Commercialization of Regenerative Medicine – Canada
Richard Maziarz, MD, Oregon Health and Science University – United States
Sunil Mehta, PhD, kSep Systems – United States
Simón Méndez-Ferrer, PhD, Spanish National Center for Cardiovascular Research (CNIC) – Spain
Michael Mendicino, PhD, Mesoblast – United States
Joseph Mierski, MSc, Hershey Medical Center – United States
William Milligan, Steminent Biotherapeutics, Inc. – Canada
C. Randal Mills, PhD, California Institute of Regenerative Medicine – United States
Stephen Minger, PhD, SLM Blue Skies Innovations Ltd. - United Kingdom
Natalie Mount, PhD, Cell Therapy Catapult – United Kingdom
Brian Murphy, Celgene – United States
Brian Newsom, Thermo Fisher Scientific, Inc. – United States
Jessie Ni, PhD, Irvine Scientific – United States
Karen Nichols, Esq. Conkwest – United States
Sarah Nikiforow, MD, PhD, Dana Farber Cancer Institute – United States
Knut Niss, PhD, Novartis Pharmaceuticals Corp. – United States
Jan Nolta, PhD, UC Davis Stem Cell Program – United States
Lynn O’Donnell, PhD, Ohio State University James Cancer Hospital – United States
Kevin O’Donnell, BioLife Solutions, Inc. – United States
Steve Oh, PhD, Bioprocessing Technology Institute – Singapore
Hideho Okada, MD, PhD, University of California San Francisco – United States
Barbara Paldus, PhD, Finesse Solutions – United States
Robert Perry, Athersys, Inc. – United States
Anne Plant, PhD, National Institute of Standards and Technology – United States
Josef Priller, MD, Charité-Universitätsmedizin Berlin – Germany
Muzaffar Qazilbash, MD, University of Texas - MD Anderson Cancer Center – United States
John Rasko, MBBS, PhD, FRCPA, FRACP, Cell & Molecular Therapies, Royal Prince Alfred Hospital – Australia
Elizabeth J. Read, MD, Ej Read Consulting LLC – United States
Katy Rezvani, MD, PhD, UT MD Anderson Cancer Center – United States
Isabelle Rivière, PhD, Memorial Sloan Kettering Cancer Center – United States
Jeff Ross, PhD, Miromatrix Medical Inc – United States
Jon Rowley, PhD, RoosterBio Inc. – United States
Joshua Schimmer, PhD, Piper Jaffray & Co. – United States
Luc Sensebé, MD, PhD, Etablissement Français Du Sang EFS – France
Mark Shannon, Quadrants Scientific Inc. – United States
Yufang Shi, PhD, Shanghai Institutes for Biological Sciences – China
Elizabeth J. Shpall, MD, MD Anderson Cancer Center – United States
Carylynn Simmons, Einhorn Yaffee Prescott Architecture & Engineering – United States
Alex Slobodianski, Technische Universität München – Germany
Ronit Slotky, PhD, Weill Cornell Medical Center – United States
Renee Smilee, MT(ASCP), Moffitt Cancer Center – United States
Robin Smith Berger, Carolinas Cord Blood Bank – United States
Jennifer Solomon, PhD, STEMCELL Technologies, Inc. – Canada
Karl Stasko, MPH, Dana Farber Cancer Institute – United States
Michele Sugrue, MT, University of Florida – United States
Keith Thompson, Cell Therapy Catapult – United Kingdom
Yuzo Toda, Fujifilm Corporation – Japan
Torsten Tonn, MD, German Red Cross Blood Donation Service East – Germany
Michael Trocchia, PE, CPIM, Novartis Pharmaceuticals Corp. – United States
Phil Vanek, PhD, GE Healthcare – United States
Viraf Vasania, Reliance Life Sciences Pvt. Ltd – India
Richard Vile, PhD, Mayo Clinic – United States
Vinod Vilivalam, West Pharmaceutical Services – United States
Sowmya Viswanathan, PhD, Centre for Commercialization of Regenerative Medicine – Canada
Kara Wacker, Foundation for the Accreditation of Cellular Therapy – United States
Phyllis Warkentin, MD, University of Nebraska Medical Center – United States
Michael Watts, PhD, MSc, Univeristy College of London Hospital – United Kingdom
Daniel J. Weiss, MD, PhD, University of Vermont College of Medicine – United States
Michael Werner, JD, Alliance for Regenerative Medicine – United States

Jennifer Wilson, MD, UCSF School of Medicine – United States
Jiwen Zhang, PhD, GE Healthcare – United States
Claudia Zylberberg, PhD, Akron Biotech – United States

Oral Abstract Presenters

Grace Asuelime, MSc, Capricor, Inc. – United States
Chaolemeng Bao, PhD, Yong Loo Lin School of Medicine, National University of Singapore – Singapore
Patrick Burger, PhD, Sanquin Research and Landsteiner Laboratory – Netherlands
Olivia Candini, PhD, University Hospital of Modena and Reggio Emilia – Italy
Raghavan Chinnadurai, PhD, Emory University – United States
Emily Culme–Seymour, PhD, London Regenerative Medicine Network – United Kingdom
Jiusheng Deng, PhD, Emory University Winship Cancer Institute – United States
Wael Abo Elkheir, MD, Cairo University – Egypt
John Fink, Brooks Automation – United States
Kristen Fousek, PhD, Baylor College of Medicine – United States
Arnaud Foussat, PhD, TeCell S.A. – France
Joseph Frank, MD, National Institute of Health – United States
Yael Gothelf, PhD, BrainStorm Cell–Therapeutics – Israel
Yanyan Han, SYZ Cell Therapy Co.– State Key Laboratory of Organ Failure Research – China
Patrick Hanley, PhD, Children’s National Medical Center – United States
Rosario Isasi, JD, McGill University – Canada
Howard Kim, PhD, Center for the Commercialization of Regenerative Medicine – Canada
Giles Kirby, University of South Australia–Mawson Institute – Australia
Selim Kuci, MD, PhD, University Children’s Hospital – Germany
Zyrafete Kuci, MD, PhD, University Children’s Hospital – Germany
Aaron Levine, PhD, MPhil, Georgia Institute of Technology – United States
Bruce Levine, PhD, University of Pennsylvania – United States
Yanling Liao, PhD, New York Medical College – United States
Shirley Mei, PhD, MSc, Ottawa Hospital Research Institute – Canada
Ian Nicoud, PhD, Fred Hutchinson Cancer Research Center – United States
Stefan Nierkens, PhD, UMC Utrecht – Netherlands
Shibani Pati, MD, PhD, Blood Systems Research Institute– University of California – United States
Helene Rouard, PharmD, PhD, Etablissement Francais du Sang Ile de France; Universite Paris–Est Creteil – France
Ruslan Semechkin, PhD, International Stem Cell Corporation – United States
Kevin Shoulars, PhD, Duke University Medical Center – United States
Philippe Willemsen, PhD, Promethera Biosciences – Belgium
Barbara Withers, MD, University of Sydney – Australia
Yi Zhang, PhD, the First Affiliated Hospital of Zhengzhou University – China
# ISCT 2015 Program

## Wednesday, May 27, 2015 Pre-Conference Day

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<th>Time</th>
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<td>0700 – 1900</td>
<td>Speaker Services</td>
<td>Genoa Room</td>
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<td>0700 – 1900</td>
<td><strong>REGISTRATION</strong></td>
<td>Promenade Foyer</td>
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<tr>
<td>0800 – 1700</td>
<td>ISCT Global Regulatory Perspectives Workshop</td>
<td>Florentine Ballroom I/II</td>
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<tr>
<td>0800 – 1500</td>
<td>ISCT Flow Cytometry Workshop</td>
<td>Florentine Ballroom III/IV</td>
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<tr>
<td>0800 – 1700</td>
<td>ISCT Cord Blood Workshop (In partnership with the <strong>new</strong> Cord Blood Association)</td>
<td>Pompeian Ballroom III/IV</td>
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<tr>
<td>0800 – 1700</td>
<td>FACT Training Workshop <strong>Pompeian Ballroom IV</strong></td>
<td>Pompeian Ballroom III/IV</td>
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<tr>
<td>1630 – 1830</td>
<td>Corporate Symposium – Miltenyi Biotec • See Corporate Guide for Details</td>
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<tr>
<td>1900 – 2130</td>
<td><strong>WELCOME ADDRESS</strong></td>
<td>Roman Ballroom</td>
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<tr>
<td>1930 – 2130</td>
<td>EXHIBIT OPEN HOUSE AND WELCOME RECEPTION</td>
<td>Exhibit Hall – Palace Ballroom</td>
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## Thursday, May 28, 2015

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<td>Promenade Foyer</td>
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<tr>
<td>0730 – 0900</td>
<td><strong>STRATEGIES FOR COMMERCIALIZATION TRACK 1 – HOT TOPICS IN PROCESS AND PRODUCT DEVELOPMENT: PARTICULATES, SERUM–FREE MEDIA, AND BIOREACTORS</strong></td>
<td>Pompeian Ballroom I/II</td>
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<td>CHAIR: OHAD KARNIELI (IL)</td>
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<td>PANELISTS:</td>
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<td>For Details on Discussion Breakdown see pg 46.</td>
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<td><em>Harvey Brandwein (US), Shannon Eaker (US), Sarah Callens (UK), Thomas Brieva (US), Eytan Abraham (IL), Dominic Clarke (US), Jerry Martin (US), Vinod Vilivalam (US), Elizabeth J. Read (US), David Fiorentini (IL), Jessie Ni (US), Andrew Campbell (US), Christopher Bravery (UK), Steve Oh (SG)</em></td>
<td>Pompeian Ballroom I/II</td>
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<tr>
<td>0800 – 0900</td>
<td><strong>TECHNICAL SESSION 1 – TECHNICAL ISSUES IN CORD BLOOD</strong></td>
<td>Florentine Ballroom I/II</td>
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<td>CHAIR: SUE ARMITAGE (US)</td>
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<td><strong>SPEAKERS:</strong></td>
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<td><em>Robin Smith Berger (US) – Cord Blood Collection 101</em></td>
<td>Florentine Ballroom I/II</td>
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<td><em>Sue Armitage (US) – How Can We Improve Cord Blood Collection Quality?</em></td>
<td>Florentine Ballroom I/II</td>
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<td><em>Karen Foster (US) – Cord Blood Collection Quality: Future Considerations</em></td>
<td>Florentine Ballroom I/II</td>
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<td>0800 – 0900</td>
<td><strong>ISCT EARLY STAGE PROFESSIONALS GROUP #ISCT_ESP – CAREER OPTIONS FOR YOUNG PROFESSIONALS: WHY MY JOB ROCKS</strong></td>
<td>Florentine Ballroom III/IV</td>
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<td>CHAIR: DEBORAH GRIFFIN (US)</td>
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<td><strong>SPEAKERS:</strong></td>
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<td><em>Patrick Hanley (US) – ESP – The Academic Cell Therapy Laboratory Director</em></td>
<td>Florentine Ballroom III/IV</td>
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<td><em>Emily Culme–Seymour (UK) – Career Options for ESPs: Leaving the Lab</em></td>
<td>Florentine Ballroom III/IV</td>
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<td><em>Saar Gill (US) – Why My Job Rocks: Translational Research in Oncology</em></td>
<td>Florentine Ballroom III/IV</td>
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<td><em>Tobi Fisher (US) – Physician Assistants: At the Forefront of Patient Care</em></td>
<td>Florentine Ballroom III/IV</td>
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</table>
### Thursday, May 28, 2015

#### QUALITY AND OPERATIONS TRACK 1 – DESIGNING A GMP FACILITY

**Chair:** Shirley Bartido (DE)

**Speakers:**
- Shirley Bartido (DE) – Design and Cost of an Academic GMP Facility
- Carylynn Simmons (US) – Designing a cGMP Facility: Perspective of the Architect in the Development and Design of a cGMP Facility

#### PLENARY SESSION 1 – PRESIDENTIAL PLENARY: DISSECTING MSC HETEROGENEITY FROM UNEXPECTED ONTOGENY TO SPECIALIZED FUNCTIONS

**Chair:** Massimo Dominici (IT)

**Speakers:**
- Ivan Martin (CH) – Use of MSC to Engineer Developmental Processes for Regenerative Medicine
- Simón Méndez-Ferrer (ES) – Bone Nature and Blood Nurture: Same Stroma?
- Kaj Fried (SE) – Glial Cells of Peripheral Nerves Give Rise to Mesenchymal Stem Cells

#### ORAL PRESENTATION SESSION 1 – MSCS

**Chair:** Luc Sensebé (FR)

**Presenters:**
- Olivia Candini (IT) – AGE RELATED miRNA SIGNATURE IN MESENCHYMAL PROGENITORS REVEALS KEY PLAYERS IN CELLULAR PERFORMANCE AND FATE
- Yanling Liao (US) – RESCUE OF THE MUCOCUTANEOUS MANIFESTATIONS IN A MOUSE MODEL OF RECESSIVE DYSTROPHIC EPIDERMOLYSIS BULLOSA (RDEB) BY HUMAN CORD BLOOD DERIVED UNRESTRICTED SOMATIC STEM CELLS (USSCS)
- Patrick Burger (NL) – FROM INDUCED PLURIPOTENT STEM CELLS TO MASSIVE ERYTHROID EXPANSION: A GLIMPSE INTO THE FUTURE OF TRANSFUSION MEDICINE
- Yael Gothelf (IL) – NUROWN™: MESENCHYMAL STEM CELLS SECRETING NEUROTROPHIC FACTORS. AN AUTOLOGOUS CELL THERAPY IN CLINICAL TRIALS FOR ALS
- Selim Kuci (DE) – COTRANSPLANTATION OF PREACTIVATED MESENCHYMAL STROMAL CELLS WITH HEMATOPOIETIC STEM CELLS IMPROVES T–CELL REGENERATION

#### ORAL PRESENTATION SESSION 2 – IMMUNOTHERAPY AND DENDRITIC CELL

**Chair:** Bruce Levine (US)

**Presenters:**
- Yi Zhang (CN) – CLINICAL STUDY OF CYTOKINE–INDUCED KILLER CELLS–BASED IMMUNOTHERAPIES IN DIFFERENT STAGES OF RENAL CELL CARCINOMA
- Barbara Withers (AU) – INFUSION OF THIRD–PARTY PARTIALLY HLA–MATCHED VIRUS–SPECIFIC T CELLS TO TREAT REFRACTORY VIRAL INFECTIONS
- Arnaud Foussat (FR) – IMMUNOTHERAPY OF NON–INFECTIOUS UVEITIS USING ANTIGEN–SPECIFIC REGULATORY T (TREG) CELLS
- Stefan Nierkens (NL) – PRECLINICAL DEVELOPMENT OF CORD BLOOD–DERIVED DENDRITIC CELL–BASED IMMUNOTHERAPIES AFTER HEMATOPOIETIC CELL TRANSPLANTATION IN CHILDREN WITH AML
- Patrick Hanley (US) – CMVPP65–SPECIFIC T CELLS GENERATED FROM NAÏVE T CELL POPULATIONS RECOGNIZE ATYPICAL BUT NOT CANONICAL EPITOPES AND MAY BE PROTECTIVE IN VIVO
### Thursday, May 28, 2015

**ORAL PRESENTATION SESSION 3 – LEGAL AND ETHICAL & QUALITY AND OPERATIONS**

**CO–CHAIRS: DEBORAH GRIFFIN (US), THOMAS LEEMHUIS (US) AND MASSIMO DOMINICI (IT)**

**PRESENTERS:**
- **Rosario Isasi (CA)** – The Role of Canadian Cell Manufacturing Facilities in Shaping the Regulatory and Commercialization Environments
- **Aaron Levine (US)** – Challenges in the Translation and Commercialization of Cellular Therapies
- **Philippe Willemsen (BE)** – Safety, Efficacy and Quality Assessment of Cell Therapeutic Products Throughout Manufacturing Changes.
- **John Fink (US)** – Protection of Innocents: Continued Sample Warm Up After Return to a Cryogenic Environment (Below –150°C) Following a Transient Ambient Picking Operation.

**Pompeian Ballroom III/IV**

**1115 – 1230**

### STRATEGIES FOR COMMERCIALIZATION TRACK 2 – ANCILLARY MATERIALS

**CO–CHAIRS: CLAUDIA ZYLBERBERG (US) AND LYNN CSONTOS (CA)**

**PANELISTS:** Jennifer Solomon (CA), Joanne Kurtzberg (US), Jiwen Zhang (US), Brian Newsom (US)

**Pompeian Ballroom I/II**

**1115 – 1230**

### ADVANCED PRACTICE PROFESSIONALS TRACK 1 – ADVANCES IN CELLULAR THERAPIES

**CHAIR: TOBI FISHER (US)**

**SPEAKERS:**
- **Helen Heslop (US)** – Novel T Cell Therapies on IND Studies
- **Katy Rezvani (US)** – Application of Natural Killer (NK) Immunotherapy in the Treatment of Cancer
- **Bambi Grilley (US)** – Novel T Cell Therapies

**Capri Room**

**1230 – 1400**

**Lunch with Exhibits**

**Exhibit Hall - Palace Ballroom**

**1245 – 1345**

**Corporate Tutorial – Irvine Scientific • See Corporate Guide for Details**

**Florentine Ballroom I/II**

**Corporate Tutorial – Thermo Fisher Scientific • See Corporate Guide for Details**

**Florentine Ballroom III/IV**

**Corporate Tutorial – Macopharma • See Corporate Guide for Details**

**Pompeian Ballroom I/II**

**Corporate Tutorial – MaxCyte • See Corporate Guide for Details**

**Pompeian Ballroom III/IV**

**1400 – 1530**

**PLENARY SESSION 2 – CELLULAR IMMUNOTHERAPY**

**CHAIR: MALCOLM BRENNER (US)**

**SPEAKERS:**
- **Malcolm Brenner (US)** – Adoptive Immunotherapy of Cancer Based on Virus–Specific T Cell
- **Richard Childs (US)** – Evaluation of Novel Pharmacologic and Genetic Approaches to Enhance Natural Killer Cell Cytotoxicity Against Cancer
- **Richard Vile (US)** – Combination of Immune Checkpoint Inhibition with Oncolytic Viroimmunotherapy

**Roman Ballroom**

**AMA PRA Category 1 Credits: 1.5**

**ACPE Credits: 1.25**

**AMA PRA Category 1 Credits: 1.25**

**ACPE Credits: 1.25**
### Thursday, May 28, 2015

#### QUALITY AND OPERATIONS TRACK 2 – MAKING THE MOST OF AUDITS AND ENSURING DATABASE ACCURACY
**Co-Chairs: Michele Sugrue (US) and Deborah Lamontagne (US)**

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<td>1400 – 1530</td>
<td><strong>QUALITY AND OPERATIONS TRACK 2 – MAKING THE MOST OF AUDITS AND ENSURING DATABASE ACCURACY</strong>&lt;br&gt;Co-Chairs: Michele Sugrue (US) and Deborah Lamontagne (US)</td>
<td>Pompeian Ballroom III/IV</td>
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<td><strong>SPEAKERS:</strong>&lt;br&gt;Michele Sugrue (US) – Developing Audits Programs and Reports&lt;br&gt;Pamela Jacobson (US) – Successful Audit Strategies to Meet Accreditation Mandates and Avoid Citation&lt;br&gt;Debra Christianson (US) – Ensuring Database Accuracy: CIBMTR’s Audit and Monitoring Program</td>
<td>Pompeian Ballroom III/IV</td>
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#### ADVANCED PRACTICE PROFESSIONALS TRACK 2 – CHRONIC GVHD: REVISED SCORING, CASE PRESENTATIONS, ADVANCES IN TREATMENT
**Chair: Kristen Kindsvogel (US)**

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<td><strong>ADVANCED PRACTICE PROFESSIONALS TRACK 2 – CHRONIC GVHD: REVISED SCORING, CASE PRESENTATIONS, ADVANCES IN TREATMENT</strong>&lt;br&gt;Chair: Kristen Kindsvogel (US)</td>
<td>Capri Room</td>
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<td><strong>SPEAKER:</strong>&lt;br&gt;Paul Carpenter (US) – Chronic GVHD: Revised Scoring, Case Presentations, Advances in Treatment</td>
<td>Capri Room</td>
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<td></td>
<td><strong>AMA PRA Category 1</strong>&lt;br&gt;Credits: 1.5&lt;br&gt;<strong>ACPE Credits: 1.5</strong></td>
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#### ADVANCED PRACTICE PROFESSIONALS TRACK 2 – CHRONIC GVHD: REVISED SCORING, CASE PRESENTATIONS, ADVANCES IN TREATMENT
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<tr>
<td>1600 – 1730</td>
<td><strong>WORKSHOP 1 – IMMUNOTHERAPY COMBINATIONS: JOINT SESSION WITH SITC</strong>&lt;br&gt;Co-Chairs: Bruce Levine (US) and Hideho Okada (US)</td>
<td>Florentine Ballroom I/II</td>
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<td><strong>SPEAKERS:</strong>&lt;br&gt;Zihai Li (US) – Taming Tregs for Adoptive T Cell Therapy of Cancer&lt;br&gt;Muzaffar Qazilbash (US) – Idiotype–KLH Primed, Activated T Cells For Adoptive Cellular Therapy of Myeloma&lt;br&gt;Hideho Okada (US) – Brain Tumor Cellular Therapy</td>
<td>Florentine Ballroom I/II</td>
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#### ADVANCED PRACTICE PROFESSIONALS TRACK 2 – CHRONIC GVHD: REVISED SCORING, CASE PRESENTATIONS, ADVANCES IN TREATMENT
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<td>1600 – 1730</td>
<td><strong>WORKSHOP 2 – APPROACHES TO IMPROVE THE DEVELOPMENT OF CELL–BASED REGENERATIVE MEDICINE PRODUCTS VIA REGULATORY SCIENCE AND ADVOCACY</strong>&lt;br&gt;Chair: Michael Mendicino (US)</td>
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<td><strong>SPEAKERS:</strong>&lt;br&gt;Michael Mendicino (US) – Cell–Based Regenerative Medicine Products for Clinical Trials – Regulatory Trends, Challenges and Opportunities&lt;br&gt;Michael Werner (US) – The Alliance for Regenerative Medicine: Regulatory Strategies for Reform&lt;br&gt;Steven Bauer (US) – The Alliance for Regenerative Medicine: Regulatory Science for Improved Characterization of Cell–Based Products: CBER’S MSC Consortium&lt;br&gt;Anne Plant (US) – The Role of Measurements and Standards in a Regulated Environment&lt;br&gt;PANELIST: Natalie Mount (UK)</td>
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#### ADVANCED PRACTICE PROFESSIONALS TRACK 2 – CHRONIC GVHD: REVISED SCORING, CASE PRESENTATIONS, ADVANCES IN TREATMENT
**Chair: Michael Mendicino (US)**

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<td>1600 – 1730</td>
<td><strong>STRATEGIES FOR COMMERCIALIZATION TRACK 3 – POTENCY AND SAFETY ASSAY DEVELOPMENT</strong>&lt;br&gt;Chair: Christopher Bravery (UK)</td>
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<td><strong>SPEAKERS:</strong>&lt;br&gt;Robert Perry (US) – Quality and Regulatory Considerations when Developing Potency Assays for Late Stage Cell Therapy Products&lt;br&gt;Claudia Zylberberg (US) – Critical Considerations for the Development of Cell Therapy Potency Assays. Case Study: Angiogenesis&lt;br&gt;Jon Rowley (US) – Assays for Developing Scalable, Cost Effective hMSC Manufacturing Processes</td>
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<td><strong>QUALITY AND OPERATIONS TRACK 3 – COMMISSIONING AND VALIDATION OF A</strong></td>
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<td><strong>CHAIR: SHIRLEY BARTIDO (DE)</strong></td>
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<td>Mark Shannon <em>(US)</em> – Clinical Manufacturing Facility Validation: “Keeping it Simple”</td>
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<td>1730 – 1900</td>
<td><strong>ADVANCED PRACTICE PROFESSIONALS TRACK 3 – NERVOUS SYSTEM REPAIR/</strong></td>
<td><strong>Capri Room</strong></td>
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<td></td>
<td><strong>PULMONARY REGENERATION</strong></td>
<td><strong>AMA PRA Category 1</strong></td>
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<td><strong>CHAIR: ALISON GULBIS (US)</strong></td>
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<td><strong>SPEAKERS:</strong></td>
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<td></td>
<td>Daniel J. Weiss <em>(US)</em> – Advances in Lung Regenerative Medicine</td>
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<td>Joanne Kurtzberg <em>(US)</em> – Game Changers: Using Cord Blood to Help the Brain</td>
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<td>1730 – 1900</td>
<td><strong>Poster Session 1</strong></td>
<td><strong>Palace Ballroom Foyer</strong></td>
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<td>1730 – 1900</td>
<td><strong>GLOBAL TO LOCAL RECEPTION</strong></td>
<td><strong>Exhibit Hall - Palace Ballroom</strong></td>
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<td>1830 – 2030</td>
<td><strong>ISCT EARLY STAGE PROFESSIONALS RECEPTION</strong></td>
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<td>0730 – 1700</td>
<td><strong>TECHNICAL SESSION 2 – GETTING PERMISSION TO USE THE CLINIIMACS</strong></td>
<td><strong>Genoa Room</strong></td>
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<td><strong>CD34–SYTEM: HOW TO CONVINCE YOUR IRB IT'S NOT RESEARCH</strong></td>
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<td><strong>CHAIR: CAROLYN KEEVER–TAYLOR (US)</strong></td>
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<td><strong>SPEAKERS:</strong></td>
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<td></td>
<td>Carolyn Keever–Taylor <em>(US)</em> – HUD Approval and Approval for Non–Research Use</td>
<td><strong>Florentine Ballroom I/II</strong></td>
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<td>Ann LeFever <em>(US)</em> – IRB Requirements To Obtain Permission Prior to the Purchase of the Clinimacs CD34–System as a HUD Usage</td>
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<td>Joanne Kurtzberg <em>(US)</em> – A Single Center Experience Obtaining Regulatory Approval for Use of the Clinimax After HUD Approval</td>
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<td>0800 – 0900</td>
<td><strong>STRATEGIES FOR COMMERCIALIZATION TRACK 4 – REIMBURSEMENT</strong></td>
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<td><strong>CHAIR: RICHARD MAZIARZ (US)</strong></td>
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<td><strong>SPEAKERS:</strong></td>
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<td></td>
<td>Stephanie Farnia <em>(US)</em> – Reimbursement Challenges and Opportunities in Hematopoietic Cell Transplant: A Case Study</td>
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<td>Panos Kefalas <em>(UK)</em> – Regional Variations in Cell Therapy Reimbursement Across the Big5EU</td>
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<td>Dawn Driscoll <em>(US)</em> – Designing Cell Therapy Trials for Regulatory and HTA Approval</td>
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<td>0800 – 0900</td>
<td><strong>QUALITY AND OPERATIONS TRACK 4 – VALIDATION BOOT CAMP PART I:</strong></td>
<td><strong>Pompeian Ballroom III/IV</strong></td>
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<td><strong>CONCEPTS OF VALIDATION AND QUALIFICATION</strong></td>
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<td><strong>CHAIR: RENE D SMILEE (US)</strong></td>
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<td><strong>SPEAKERS:</strong></td>
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<td></td>
<td>Lizette Caballero <em>(US)</em> – Qualification, Validation and Verification: Say it Again?</td>
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PLENARY SESSION 3 – ADVANCED PHASE CLINICAL TRIAL UPDATES IN CELL BASED THERAPIES
CHAIR: ROBERT DEANS (US)

SPEAKERS:
- Robert Deans (US) – MultiStem®: Clinical Development Experience in Ischemic Injury and Inflammatory Disease
- Jeffrey Cohen (US) – Mesenchymal Stem Cell Transplantation to Treat Multiple Sclerosis

QUALITY AND OPERATIONS TRACK 5 – VALIDATION BOOT CAMP PART II: EQUIPMENT AND SUPPLIES
CHAIR: LYNN O’DONNELL (US)

SPEAKERS:
- Emily Hopewell (US) – Validation of an X–Ray Irradiator to Replace a Cesium Irradiator
- Ronit Slotky (US) – The Dextran Shortage – How to Approach the Validation of a New Critical Reagent
- Panel Members and Audience – Moving Your Facility – Qualification, Verification, Validation? All of the Above (And Then Some)

WORKSHOP 3 – STEM CELLS FOR NEUROLOGICAL DISORDERS
CHAIR: JOSEF PRILLER (DE)

SPEAKERS:
- Josef Priller (DE) – Stem Cells for Brain Repair
- Jan Nolta (US) – Mesenchymal Stem/Stromal Cells Engineered to Produce Brain–Derived Neurotrophic Factor as a Potential Treatment for Huntington’s Disease
- Chotima Bötcher (DE) – Hematopoietic Progenitors as a Tool to Treat Neurodegenerative Disorders?

ORAL PRESENTATION SESSION 4 – TRANSLATIONAL PROCESS DEVELOPMENT
CHAIR: OHAD KARNIELI (IL)

PRESENTERS:
- Ian Nicoud (US) – INTEGRATED AUTOMATION OF MULTIPLE UNIT OPERATIONS FOR PROCESSING AND EXPANDING CORD BLOOD HEMATOPOIETIC STEM AND PROGENITOR CELLS
- Raghavan Chinnadurai (US) – REPLICATIVE SENESCENCE ASSOCIATED IMPAIRMENT OF IMMUNOSUPPRESSIVE PROPERTIES OF MESENCHYMAL Stromal CELLS
- Howard Kim (CA) – PROCESS DEVELOPMENT TOWARDS EFFICIENT LARGE–SCALE MANUFACTURING OF AN ALOGENIC NATURAL KILLER CELL THERAPY
- Yanyan Han (CN) – A NOVEL CANCER IMMUNOTHERAPY WITH MULTIPLE TUMOR ANTIGEN ACTIVATED AUTOLOGOUS T CELLS FOR HEPATOCELLULAR CARCINOMA
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<tr>
<td>1115 – 1230</td>
<td><strong>ORAL PRESENTATION SESSION 5 – CELL AND GENE THERAPY AND</strong></td>
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<td><strong>HEMATOPOIETIC STEM CELLS</strong></td>
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<td><strong>CO–CHAIRS: HELEN HESLOP (US) AND ELIZABETH J. SHPALL (US)</strong></td>
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<td><strong>PRESENTERS:</strong></td>
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<td>Kristen Fousek (US) – SAFETY OF MULTIPLE DOSES OF CAR T CELLS</td>
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<td>Bruce Levine (US) – CHIMERIC ANTIGEN RECEPTOR MODIFIED T CELLS</td>
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<td>DIRECTED AGAINST CD19 (CTL019) INDUCE CLINICAL RESPONSES IN</td>
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<td>PATIENTS WITH RELAPSED OR REFRACTORY CD19+ LYMPHOMAS</td>
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<td>Wael Abo Elkheir (EG) – COMBINED LOCAL MELANOCYTES AND SYSTEMIC</td>
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<td>MESENCHYMAL STEM CELL INJECTION IN VITILIGO TREATMENT</td>
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<td>Jiusheng Deng (US) – FUSOKINE GIFT4 TRIGGERS NOVEL B CELL FUNCTION</td>
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<td>ON HEMATOPOIETIC STEM CELLS</td>
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<td>Kevin Shoulars (US) – ALDEHYDE DEHYDROGENASE, BUT NOT VIABILITY</td>
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<td>OF CD34 CELLS, PREDICTS POTENCY AND ENGRAFTMENT AFTER CORD</td>
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<td>BLOOD TRANSPLANTATION</td>
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<td><strong>STRATEGIES FOR COMMERCIALIZATION TRACK 5 – CELL THERAPY</strong></td>
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<td><strong>BIOLOGISTICS: OPTIMIZING COST IN A COLD SUPPLY CHAIN</strong></td>
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<td><strong>CHAIR: WILLIAM MILLIGAN (CA)</strong></td>
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<td><strong>SPEAKERS:</strong></td>
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<td>Kevin O’Donnell (US) – Good Cold Chain Distribution Practice Considerations</td>
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<td>for Planning and Designing Cost–Effective Cold–Chain Strategies</td>
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<td>Brian Murphy (US) – Impact of Cold Chain Decisions on Critical Quality</td>
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<td>Attributes and Cost of Goods</td>
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<td>Michael Trocchia (US) – Logistic Requirements for Cell Therapy Products and How These Impact Cost</td>
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<tr>
<td>1230 – 1400</td>
<td><strong>Lunch with Exhibits</strong></td>
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<td>1230 – 1400</td>
<td><strong>ISCT PRESIDENTIAL TASK FORCE ON THE USE OF UNPROVEN CELLULAR</strong></td>
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<td><strong>THERAPIES ROUND TABLE: CONNECTING OUR STAKEHOLDERS,</strong></td>
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<td><strong>COMMUNICATING KNOWLEDGE, TRANSLATING THE PROVEN</strong></td>
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<td><strong>CO–CHAIRS: MASSIMO DOMINICI (IT), KAREN NICHOLS (US), AARON</strong></td>
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<td><strong>LEVINE (US), AND JOHN RASKO (AU)</strong></td>
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<td><strong>Speakers: ISCT Presidential Task Force Members</strong></td>
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<td>1245 – 1345</td>
<td><strong>Corporate Tutorial – Fresenius Kabi • See Corporate Guide for Details</strong></td>
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<td>1245 – 1345</td>
<td><strong>Corporate Tutorial – Pall Life Sciences • See Corporate Guide for Details</strong></td>
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<td>1245 – 1345</td>
<td><strong>Corporate Tutorial – BioLife Solutions • See Corporate Guide for Details</strong></td>
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<td>1245 – 1345</td>
<td><strong>Corporate Tutorial – EMD Millipore • See Corporate Guide for Details</strong></td>
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PLENARY SESSION 4 – GAME CHANGERS: POLICY AND PRACTICE CHANGES
ENABLING THE COMMERCIALIZATION OF CELL AND TISSUE PRODUCTS
CHAIR: ABY J. MATHEW (US)

SPEAKERS:
- Nick Crabb (UK) – The Health Technology Assessment and Reimbursement of Cell Therapies
- Reni Benjamin (US) – Investing and Enabling the Cell Therapy Space: A Perspective from Wall Street

QUALITY AND OPERATIONS TRACK 7 – ENSURING QUALITY IN CELL BASED CLINICAL TRIALS
CHAIR: VIRAF VASANIA (IN)

SPEAKERS:
- Viraf Vasania (IN) – Ensuring Quality in Stem Cell–Based Products for Multi–Centric Clinical Trials
- Stephen Minger (UK) – Stem Cell Medicine– Its Potential and How to Ensure Quality in Multiple Trials Across Different Geographies
- Soumya Viswanathan (CA) – Product Release Testing Considerations for Directed Allogeneic or Autologous Products
- Aisha Khan (US) – Clinical Trials: Moving Cell Based Therapies to the Clinic

WORKSHOP 4 – CELL BASED ASSAYS AS POTENCY RELEASE CRITERIA FOR MSC: JOINT SESSION WITH IFATS
CHAIR: LUC SENSEBÉ (FR)

SPEAKERS:
- Christian Jorgensen (FR) – MSC Based Therapy for Severe Osteoarthritis of the Knee: the ADIPOA Experience
- Mauro Krampera (IT) – Immunological Assays as Potency Release Criteria for Mesenchymal Stromal Cells (MSCs)
- Jeffrey Gimble (US) – Adipose Based Cell Assays

WORKSHOP 5 – ADVANCES IN CELL THERAPIES AND REGENERATIVE MEDICINE IN PULMONARY DISEASES AND CRITICAL ILLNESSES
CHAIR: DANIEL J. WEISS (US)

SPEAKERS:
- Jennifer Wilson (US) – Mesenchymal Stem Cells for the Treatment of ARDS: Results of a Phase 1 Clinical Trial
- Daniel J. Weiss (US) – It’s not Just MSCs: Other Adult Stem Cell Populations for Use in Cell Therapies for Lung Diseases and Critical Illnesses
- Sarah Gilpin (US) – Lung Bioengineering and Regeneration Based on Native Lung Scaffolds

STRATEGIES FOR COMMERCIALIZATION TRACK 6 – FINANCING AND INVESTMENT
CHAIR: RENI BENJAMIN (US)

SPEAKERS:
- Reni Benjamin (US) – The Declaration of Investment: Not All Cell Therapies are Created Equal
- Joshua Schimmer (US) – Investor’s Views of the Emerging GenomeRx Revolution
- Jason Kolbert (US) – Paradigms in Healthcare – Could the “Stem Cell Space” Be Next?
## Friday, May 29, 2015

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<tr>
<td>1600 – 1730</td>
<td><strong>QUALITY AND OPERATIONS TRACK 8 – RATIONALE AND CHALLENGES OF BUILDING A GMP FACILITY IN ACADEMIA: A ROUND TABLE DISCUSSION</strong>&lt;br&gt;<strong>CHAIR:</strong> SHIRLEY BARTIDO (DE)&lt;br&gt;<strong>SPEAKERS:</strong>&lt;br&gt;Keith Thompson (UK) – Perspective on Cell Therapy Catapult Facility&lt;br&gt;Adrian Gee (US) – Perspective on Baylor College of Medicine Facility&lt;br&gt;Isabelle Rivière (US) – Perspective on Memorial Sloan Kettering Cancer Center Facility</td>
<td>Pompeian Ballroom III/IV</td>
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<td>1730 – 1900</td>
<td><strong>Poster Session 2</strong></td>
<td>Palace Ballroom Foyer</td>
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<td>1900 – Midnight</td>
<td><strong>GALA DINNER • Ticketed Event - See Registration Desk For Details</strong></td>
<td>Roman Ballroom</td>
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<tr>
<td>0730 – 1600</td>
<td><strong>Speaker Services</strong></td>
<td>Genoa Room</td>
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<tr>
<td>0730 – 1500</td>
<td><strong>REGISTRATION</strong></td>
<td>Promenade Foyer</td>
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<td>0800 – 0900</td>
<td><strong>TECHNICAL SESSION 3 – TECHNICAL TOOLS OF IMMUNOTHERAPY: EXPANSION OF NON-ANCHORED CELLS</strong>&lt;br&gt;<strong>CHAIR:</strong> PAUL ELDRIDGE (US)&lt;br&gt;<strong>SPEAKERS:</strong>&lt;br&gt;Natalia Lapteva (US) – Large Scale Manufacturing of Clinical–Grade Natural Killer Cells&lt;br&gt;Isabelle Rivière (US) – Engineering CAR–T cells for Cancer Immunotherapy&lt;br&gt;Tosten Tonn (DE) – Clinical Scale Isolation and Expansion of Untouched Antigen Specific T Cells</td>
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<td>0915 – 1045</td>
<td><strong>PLENARY SESSION 5 – TISSUE ENGINEERING</strong>&lt;br&gt;<strong>CHAIR:</strong> ALLAN DIETZ (US)&lt;br&gt;<strong>SPEAKERS:</strong>&lt;br&gt;Jeff Ross (US) – Advances in Natural Scaffolds to Grow Transplantable Organs&lt;br&gt;Scott Hollister (US) – 3D Printing for Patient Specific Implants and Regenerative Medicine&lt;br&gt;Utkan Demirci (US) – Advanced Technologies in Bioprinting and Biofabrication On–Chip Tissues Models</td>
<td>Roman Ballroom&lt;br&gt;AMA PRA Category 1 Credits: 1.5</td>
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<td>0915 – 1045</td>
<td><strong>QUALITY AND OPERATIONS TRACK 9 – THE GOLDEN THREAD OF QUALITY</strong>&lt;br&gt;<strong>CO-CHAIRS:</strong> ALEX SLOBODIANSKI (DE) AND KARA WACKER (US)&lt;br&gt;<strong>SPEAKERS:</strong>&lt;br&gt;Alex Slobodianski (DE) – Quality Aspects of ATMP Development in Europe&lt;br&gt;Bangon (Day) Longsomboon (US) – Regulatory Expectations for Quality Management in the U.S.&lt;br&gt;Phyllis Warkentin (US) – Using the FACT Common Standards as a Stepping Stone</td>
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<td>1045 – 1115</td>
<td>Coffee Break with Exhibits</td>
<td>Exhibit Hall - Palace Ballroom</td>
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#### Oral Abstracts Session 6 – Regenerative Medicine and Tissue Engineering

**Chair:** Jacques Galipeau (US)

**Presenters:**
- **Giles Kirby** (AU) – Delivering a Cell Therapy
- **Emily Culme-Seymour** (UK) – Cost of Stem Cell-Based Tissue-Engineered Airway Transplants in the UK: Case Series
- **Chaolemeng Bao** (SG) – Pre-Transplant Osteogenic Induction Promotes Primitive Mesenchymal Stem Cells’ Functional Secretion and Healing Efficacy
- **Joseph Frank** (US) – Enhanced Stem Cell and Progenitor Cell Homing Following IV Injection to Skeletal Muscle in a Murine Model of Muscular Dystrophy Induced by Pulsed Focused Ultrasound (PFUS)

#### Oral Abstracts Session 7 – Cardiovascular Regeneration and Nervous System Repair

**Chair:** Josef Priller (DE)

**Presenters:**
- **Grace Asuelime** (US) – Process Development of Therapeutic Exosome Manufacturing Process to Create a Non-Living Regenerative Medicine Product
- **Shirley Mei** (CA) – Efficacy of Intramuscular Administration of Placenta-Derived Mesenchymal-Like Adherent Stromal Cells (PLX-PAD) in Monocrotaline-Induced Pulmonary Arterial Hypertension
- **Ruslan Semechkin** (US) – Human Parthenogenetic Derived Neural Stem Cells for the Treatment of Parkinson’s Disease
- **Shibani Pati** (US) – Wnt3a Recapitulates the Neuroprotective Effects of Mesenchymal Stem Cells and Promotes Neurocognitive Recovery in Traumatic Brain Injury

#### Quality and Operations Track 10 – Technology Transfer: Academia to Industry (And Back Again)

**Chair:** Mark Lowdell (UK)

**Speaker:**
- **Moya Daniels** (US) – Technological Development of a Cellular Therapeutic: Industry to Academia and Back Again
- **Mark Lowdell** (UK) – Moving a Trial from Academic GMP to CMO cGMP – a Real World Experience in Crossing the Atlantic

#### Cytotherapy: How to Review Papers for Peer-Reviewed Journals

**Chair and Speaker:** John Barrett (US)

#### ISCT Annual General Meeting

**Feature Presentation by NASA: Stem Cell-Based Tissue Regenerative Health in Microgravity**

**Presenter:**
- **Eduardo Almeida, PhD**, NASA Research Scientist, Co-Director, Bone and Cell Signaling Laboratory, Space Biosciences Division, NASA Ames Research Center (US)
Saturday, May 30, 2015

WORKSHOP 6 – IMMUNOMODULATION
CHAIR: YUFANG SHI (CN)

SPEAKERS:
- Yufang Shi (CN) – Stem Cells Immunology and Pathological and Therapeutic Implications
- Katarina Le Blanc (SE) – Bringing Mesenchymal Stem Cells into the Clinic
- Francesco Dazzi (UK) – Myeloid Precursors as Targets of Mesenchymal Stromal Cell Therapeutics

Florentine Ballroom I/II

STRATEGIES FOR COMMERCIALIZATION TRACK 8 – STEM CELL BIOPROCESSING/MANUFACTURING TECHNOLOGIES
CHAIR: KNUT NISS (US)

SPEAKERS:
- Anthony Davies (US)
- Steve Oh (SG) – Serum–Free Media Requirements Differ Greatly Between Monolayer and Microcarrier Cultures
- Sunil Mehta (US) – Overcoming Challenges of Volume Reduction and Cell Washing Process in Cell Therapy Manufacturing

Pompeian Ballroom I/II

QUALITY AND OPERATIONS TRACK 11 – STAFFING MODELS AND SUCCESSION PLANNING
CHAIR: KARL STASKO (US)

SPEAKERS:
- Sarah Nikiforow (US) – Challenges in Preparing Cellular Therapy Labs and Staffing for an Ever–Changing Future
- Suzanne Dworsky (US) – Staffing Models and Succession Planning for the Cell Therapy Laboratory
- Joseph Mierski (US) – Results from the ISCT Laboratory Practices Committee Cell Therapy Laboratory Staffing Survey

Pompeian Ballroom III/IV

1515 – 1530
Coffee Break

Exhibit Hall - Palace Ballroom

1530 – 1535
Announcement: Best Oral Abstract Award/Best Poster Award Recipients

Roman Ballroom

PLENARY SESSION 6 – NOT–FOR–PROFIT ORGANIZATIONS AND CONTRIBUTIONS TO ADVANCING CELL THERAPIES
CHAIR: C. RANDAL MILLS (US)

SPEAKERS:
- C. Randal Mills (US) – The California Institute for Regenerative Medicine
- Michael May (CA) – CCRM – Canada’s RM Commercialization Vehicle: Progress and Status after Four Years of Bridging the Gap
- Keith Thompson (UK) – Accelerating the Growth of the Industry by Supporting Commercialization

Roman Ballroom

AMA PRA Category 1 Credits: 1.5

QUALITY AND OPERATIONS TRACK 12 – TECHNICAL TOWN HALL MEETING: DISCUSSION OF COMMON OR EMERGING ISSUES FACING CELL THERAPY FACILITIES
CHAIR: VICKI ANTONENAS (AU)

PANELISTS: Paul Eldridge (US), Michael Watts (UK), Heather Daley (US), Renee Smilee (US)

Florentine Ballroom III/IV
Plenary Session Summaries

PLENARY SESSION 1 – PRESIDENTIAL
PLENARY ON DISSECTING MSC HETEROGENEITY FROM UNEXPECTED ONTOGENY TO SPECIALIZED FUNCTIONS

THURSDAY, MAY 28TH, 2015 • TIME: 0915 – 1045
Roman Ballroom

Chair: Massimo Dominici

Since the early findings on MSC isolation, it has been hypothesized that these progenitors may have an intrinsic heterogeneity. After decades, we progressively understand that a lack of an in-depth comprehension of this heterogeneity could be negatively impacting the outcome of regenerative medicine approaches based on MSC. We are also aware that heterogeneity may be dependent from the MSC tissue sources, be influenced by the ex-vivo culture conditions and be driven by the use of biomaterials, if applicable. Thus, a precise understanding of the different MSC sub-types and their precise functions in vivo is demanded to favour a more robust introduction of the selected cell population into specialized functions to be restored. In this session we will dissect the ontogeny of MSC proposing novel insights on their origins and how these evidences could be impacting normal development/omeostasis and regenerative processes in the adult mammalian body. Starting from these more basic concepts, then speakers will also discuss scalability, process control and regulatory compliance in manufacturing cell-based products.

USE OF MSC TO ENGINEER DEVELOPMENTAL PROCESSES FOR REGENERATIVE MEDICINE
Ivan Martin

Following the exemplifying context of cartilage and bone regeneration, this lecture will describe and discuss alternative approaches to evolve classical tissue engineering paradigms towards possibly more effective grafts, with the potential for a broader clinical use. The lecture will then propose and discuss the concept of engineering regenerative strategies by recapitulating developmental processes, exploiting the own body as the in vivo bioreactor. Rather than engineering a tissue, the strategy targets the use of cells (e.g., MSC) to engineer the different stages of a process which recapitulate events of development (e.g., endochondral ossification). The product will be a tissue containing all necessary and sufficient cues to remodel into the target repair tissue upon grafting. The perspective will also address issues related to scalability, process control and regulatory compliance in manufacturing cell-based products and highlight the need not only to automate, but also to streamline and simplify typical production processes.

BONE NATURE AND BLOOD NURTURE: SAME STROMA?
Simón Méndez-Ferrer

Contrasting the relatively well known hierarchy and roles of haematopoietic cells in the bone marrow, lineage and functional relationships among non-hematopoietic cells have remained elusive, despite the large interest raised by their haematopoietic and immune regulatory properties. Among other cell types, endothelial cells, neuroglial cells, mesenchymal progenitors and osteochondral cells have been proposed as key elements of the haematopoietic stem cell (HSC) niche in the bone marrow. However, the developmental and functional relationships of these cells have remained poorly characterised. Technological developments have allowed start dissecting them but have also evidenced limitations that need to be overcome in order to advance the field. We previously showed that bone marrow nestin+ cells innervated by sympathetic nerve fibers regulate normal haematopoietic stem cells. Our recent data has demonstrated that this circuitry is critically damaged in myeloproliferative neoplasms, diseases that were previously thought autonomously driven by mutated haematopoietic stem cells. We will discuss origins and functions of bone marrow mesenchymal stem cells (MSCs) and the potential therapeutic implications.

GLIAL CELLS OF PERIPHERAL NERVES GIVE RISE TO MESENCHYMAL STEM CELLS
Kaj Fried

The early glia of the peripheral nervous system, the Schwann cell precursors (SCPs) can be considered as multipotent stemlike cells. We have shown that the peripheral parasympathetic ganglia in mice arise from glial cells in nerves, not migrating neural crest. This mechanism explains both how parasympathetic ganglia are formed and how they are wired. SCPs can also generate MSCs. We have demonstrated that sensory nerve SCPs detach and contribute with MSCs that give rise to pulp cells and matrix-producing odontoblasts during development of teeth. This unanticipated mechanism operates both during embryogenesis and during the continuous growth of the mouse incisor during adulthood. The medical implication of this novel aspect of how soft and mineralized tissue are generated includes
better understanding of growth mechanisms, providing new approaches to regeneration and restoration following trauma. Consequently, the SCPs could represent a significant cell source for regenerative medicine and cell therapy.

PLENARY SESSION 2 – CELLULAR IMMUNOTHERAPY

THURSDAY, MAY 28TH, 2015 • TIME: 1400 – 1530

Chair: Malcolm Brenner

In this session we will illustrate how three convergent technologies have the potential to profoundly increase the success of cancer. Oncolytic viruses have shown promise as single agent treatment of malignant disease but have limited systemic effect and are rarely curative. Adoptive lymphocyte therapies have been curative in some disorders but are limited by tumor immune evasion strategies while antibodies that override immune checkpoints can boost intrinsic innate immunity but may have limited potency for most tumors as single agents. We will show how combinations of these clinically tested approaches can prove highly synergistic and offer us the potential for broadly successful future applications.

ADOPTIVE IMMUNOTHERAPY OF CANCER BASED ON VIRUS–SPECIFIC T CELLS
Malcolm Brenner

Adoptive transfer of chimeric antigen expressing T cells (CAR-T cells) has proved an extraordinarily potent treatment of some hematological malignancies. Extension to solid tumors has been handicapped by lack of tumor specific targets, tumor immune evasion strategies and incomplete activation (or co-stimulation) of the CAR-expressing T cells, reducing their expansion and persistence in vivo. This presentation shows how virus specific T cells can be adapted to overcome these limitations and to discuss fruitful combination with oncolytic viruses and immune checkpoint antibodies.

EVALUATION OF NOVEL PHARMACOLOGIC AND GENETIC APPROACHES TO ENHANCE NATURAL KILLER CELL CYTOTOXICITY AGAINST CANCER
Richard Childs

Drug and cell-based therapies developed to bolster humoral and T cell immunity represents an established and growing component of cancer therapeutics. Although NK cells have long been known to have advantages over T cells in terms of their capacity to induce antigen independent host immune responses against malignancies, their therapeutic potential in the clinic has gone largely unexplored. Given their rapid and efficient method of recognizing tumor cells, which is distinct from other lymphocyte populations, NK cells represent a unique immune cell, which if utilized successfully, could improve the outcome of cell based cancer immunotherapy. Here we will review a number of different pharmacological and genetic methods to bolster NK cell anti-tumor immunity based on modifying pathways that activate or suppress NK cell function as well as methods to sensitize tumors to NK cell cytotoxicity. We will also review advances in our ability to expand NK cells ex vivo and manipulate their capacity to home to the tumor which now provide new methods for investigators to harvest the potential of NK cell-based cancer immunotherapy in the clinic.

COMBINATION OF IMMUNE CHECKPOINT INHIBITION WITH ONCOLYTIC VIROIMMUNOTHERAPY
Richard Vile

Oncolytic viruses can lead to significant tumor regressions. We, and others, have shown that these therapeutic effects are often dependent upon immune reactivities stimulated by the virus infection. As a result, we have used the oncolytic Vesicular Stomatitis Virus (VSV) as a platform for expression of multiple tumor associated antigens (TAA). We have shown that systemic treatment of mice bearing established tumors with VSV-TAA is effective against melanoma, prostate and gliomas. In most cases, anti tumor efficacy is associated with a Th17 T cell response against a variety of self TAA encoded by the virus. In order to boost these anti tumor T cell responses, we have combined VSV-TAA therapy with immune checkpoint inhibitor therapy. With careful timing of virus and checkpoint inhibitor antibody treatment, we have shown that suboptimal VSV-TAA therapy can be significantly enhanced with both anti-PD1 and anti-CTLA4 treatments. Interestingly, immune checkpoint inhibition is associated with the de-repression of T cell responses against TAA which are not observed with the VSV-TAA therapy alone.

PLENARY SESSION 3 – ADVANCED PHASE CLINICAL TRIAL UPDATES IN CELL BASED THERAPIES

FRIDAY, MAY 29TH, 2015 • TIME: 0915 – 1045

Chair: Robert Deans

Excitement and momentum are building in cell therapy with achievements in gene engineering and immunotherapies combined with advancement of a number of adherent cell
therapies through clinical proof of mechanism. Case studies will be presented which highlight clinical data and development approaches in these platforms, emphasizing gene therapy for beta-thalassemia, multiple sclerosis, stroke and ulcerative colitis. Discussion will range from progress and challenges in cell processing and delivery to interpreting clinical data in light of hypotheses for mechanism of action. Presenters will discuss remaining challenges in development including bedside to bench efforts in interpretation of clinical bioassay information and refining the clinical design approach.

MULTISTEM®: CLINICAL DEVELOPMENT EXPERIENCE IN ISCHEMIC INJURY AND INFLAMMATORY DISEASE
Robert Deans

MultiStem® is an adult adherent allogeneic cell product in mid-phase clinical development; acute MI, ARDS and two recently accrued Phase II studies in ulcerative colitis and ischemic stroke. Early clinical data in acute MI and allogeneic transplant have been published and supported therapeutic hypotheses in immunomodulation and angiogenesis. Top line clinical reports from the Phase II ulcerative colitis study did not show clinical benefit; clinical design and dose parameters will be discussed relative to patient bioassays and mechanism. Top line clinical readout from a Phase II stroke study indicated that the primary composite endpoints was not met. MultiStem treatment was associated with lower rates of mortality and life threatening adverse events. Post-hoc analysis shows that patients who received MultiStem treatment earlier in the treatment window had more robust recovery rates in comparison to placebo and relative to patients who received later MultiStem treatment. Analysis of these data and therapeutic mechanism will be discussed.

MESENCHYMAL STEM CELL TRANSPLANTATION TO TREAT MULTIPLE SCLEROSIS
Jeffrey Cohen

Mesenchymal stem cells (MSCs) have potent immunomodulatory, tissue-protective, and repair-promoting properties in vitro and in animal models. Clinical trials support the safety and efficacy of MSC transplantation in several human conditions. Published experience in multiple sclerosis (MS) is modest. We recently completed a phase 1 study of autologous MSC transplantation in MS. 1-2x10^6 culture-expanded, bone-marrow-derived MSCs/kg were administered IV to 16 women and 8 men, 10 relapsing-remitting and 14 secondary progressive, mean age 46.5 and EDSS 5.2, and 25% with Gd-enhancing brain lesions. Mean cell dosage (requiring 1-3 passages) was 1.9x10^6 MSCs/kg (range 1.3-2.0) with post-thaw viability >95%. Cell infusion was well tolerated. There were no treatment-related severe or serious adverse events, or indication of disease activation.

Trend analysis using splines suggested benefit in a number of exploratory clinical, imaging, and laboratory assessments, which will help guide planned phase 2 testing.

DOWN UNDER WHERE? OVERCOMING THE CHALLENGES OF AN INTERNATIONAL MULTICENTRE B-THALASSEMIA MAJOR STUDY OF LENTIGLOBIN BB305-GENE-MODIFIED AUTOLOGOUS CD34+ CELLS WITH CENTRALISED MANUFACTURING
John Rasko


The Phase 1/2 Northstar (HGB-204) Study is designed to evaluate the feasibility, safety and efficacy of LentiGlobin (BB305) drug product in the treatment of subjects with beta-thalassemia major. The study has enrolled fifteen transfusion-dependent subjects who will be evaluated for safety and efficacy post-myeloablative transplant (clinicaltrials.gov NCT01745120). Transplantation with autologous CD34+ cells transduced with a replication-defective, self-inactivating LentiGlobin BB305 lentiviral vector containing an engineered β-globin gene (βAT87Q) resulted in early reduction or elimination of transfusion requirements in subjects treated in the ongoing HGB-204 and 205 studies. To date, all AEs are consistent with myeloablative conditioning and no AEs were considered to be related to drug product, including no replication competent lentivirus (RCL). As of 1 December 2014, seven subjects have undergone gene-therapy via infusion of transduced CD34+ cells. All four subjects, with >3 months of follow up, are producing significant βA-T87Qglobin and are transfusion independent after an overall median follow up of 94 days (range: 21-372 days). Details of patient eligibility, vector design, efficiency of transduction and safety parameters (insertion site assessment and RCL analysis) for gene therapy of thalassemia will be provided.

In this presentation we draw attention to the challenges of undertaking and co-ordinating an international multicentre study of this scope. In particular we provide examples that emphasise the need for a robust quality risk management system, rapid and clear international communication, as well as the prospective provision of adequate information to effectively perform duties, fulfil local regulatory requirements and recruit local knowledge and experience.
PLENARY SESSION 4 – GAME CHANGERS: POLICY AND PRACTICE CHANGES ENABLING THE COMMERCIALIZATION OF CELL AND TISSUE PRODUCTS

FRIDAY, MAY 29TH, 2015 • TIME: 1400 – 1530

Roman Ballroom

Chair: Aby J. Mathew

The development of cell therapies and regenerative medicine has reached a transitional period. There have been promising (and disappointing) clinical results, a surge in investment from financial markets and pharmaceutical partners, and further development of manufacturing and commercialization models. As a greater number of new therapies progress closer towards approval and commercialization, there has also been further discussion (and concern) regarding the reimbursement models and the regulatory approval pathways. This session will discuss several key aspects that are of interest to commercialization of these therapies, and will supplement the clinical highlights presented in other sessions. This Plenary session will share perspectives on reimbursement, finance/investing, and also the conditional regulatory approval pathway in Japan.

THE HEALTH TECHNOLOGY ASSESSMENT AND REIMBURSEMENT OF CELL THERAPIES
Nick Crabb

The presentation will briefly introduce the evaluation and decision frameworks used in the NICE Technology Appraisals Programme before considering the suitability of these frameworks for cell therapy products. Challenges for both the developers of cell therapy products and those charged with their evaluation and reimbursement will then be presented. There will also be coverage of on-going work recommended by the UK regenerative medicine expert group and a summary of activities at NICE to support managed access to high value health technologies.

INVESTING AND ENABLING THE CELL THERAPY SPACE: A PERSPECTIVE FROM WALL STREET
Reni Benjamin

The cell therapy space has rapidly advanced from relative obscurity to the forefront of next generation therapeutics addressing areas of large unmet need. The “driving force” for the advancement of the future of cell therapy remains biotechnology companies willing to take large risks. In our view, the “fuel” to drive the development of innovative products remains scientific knowledge and investor dollars. However, Wall Street remains both enamored and skeptical at the same time, selectively investing in those companies with robust clinical data with a clear regulatory path to commercialization. This presentation will address the good, the bad, and the ugly aspects of the cell therapy universe from the point of view of Wall Street including an understanding of the investor mindset, the relative valuation metrics and factors that drive market value, the variety of investors involved, as well as the potential to “time” the market for funding opportunities.

NEW REGENERATIVE MEDICINE DEVELOPMENT IN JAPANESE INDUSTRY
Yuzo Toda

This presentation will cover updates of Japanese development circumstances of Regenerative Medicine and Cell Therapy. Additionally, this presentation will explore FIRM (Japanese Industry Association of Regenerative Medicine) and its activities especially for supporting foreign companies to develop regenerative medicine and cell therapy products in Japan recently.

PLENARY SESSION 5 – TISSUE ENGINEERING

SATURDAY, MAY 30TH, 2015 • TIME: 0915 – 1045

Roman Ballroom

Chair: Allan Dietz

The goal of tissue engineering is to produce new or improved tissue consisting of cells (sometimes multiple cell types) and matrix. This session will examine multiple approaches to produce or use matrix and the benefits and potential of each approach. Decellularized whole organs provide the most native and complex matrix available. Dr. Jeff Ross from Miromatix will discuss efforts to use all or parts of decellularized organs for tissue engineering. In some cases, only segments of tissue or organs need repair. Dr. Scott Hollister from the University of Michigan will discuss his experience using patient specific 3D printing to produce matrix as scaffold for tissue engineering. Finally, Dr. Utkan Demirci from Stanford will look at the application of micro and nano scale solutions to drive ‘bottom up’ tissue engineering. Together, these speakers will discuss a variety of the latest approaches to tackle some of the toughest medical problems.

ADVANCES IN NATURAL SCAFFOLDS TO GROW TRANSPLANTABLE ORGANS
Jeff Ross

In the United States alone, more than 17,000 people are currently awaiting a liver transplant. The demand for a transplant far exceeds the approximately 6,500 livers that are
available annually, as there are no existing treatments capable of long-term restoration of liver function except transplantation. To address this critical need, we are developing a perfusable, revascularized liver graft to treat chronic liver failure. The liver graft is based on perfusion decellularization technology capable of creating a scaffold of ECM that is biologically complex, architecturally correct, and that retains the native environmental cues that are essential for successful revascularization.

3D PRINTING FOR PATIENT SPECIFIC IMPLANTS AND REGENERATIVE MEDICINE
Scott Hollister

The past two years have seen an explosion in the application of 3D printing for clinical applications. The vast majority of these applications have been for surgical/procedure planning. However, a natural extension of these applications is to create patient specific implants and subsequently, biologic implants that integrate 3D printed materials with biologics, possibly 3D printed biologics. While a number of research groups are pursuing these goals, clinical transition of 3D printed implants and biologic devices remains sparse. We will describe our current work transitioning 3D printed implants and biologic devices (3D printed materials delivery cells and/or growth factors) from pre-clinical investigations to patient use, both the breakthroughs and setbacks. These include applications to treat tracheobronchomalacia and periodontal disease using 3D printed bioresorbable polymers. Finally, the regulatory (especially quality control for patient specific devices) and model implementation issues for 3D printing of patient specific implants and biologic devices will be discussed.

ADVANCED TECHNOLOGIES IN BIOPRINTING AND BIOFABRICATION ON-CHIP TISSUE MODELS
Utkan Demirci

The natural microenvironment of native tissues are composed of extracellular matrix (ECM), blood vasculature, and supporting stromal cells. The physical characteristics of ECM as well as the cellular components play a vital role in controlling cell proliferation, apoptosis, metabolism, and differentiation. To reproduce the natural microenvironment outside the human body for drug testing and experimental biology, two-dimensional (2-D) and murine models are routinely used. Although these conventional approaches are employed in basic and preclinical studies, they still present challenges. The three-dimensional (3-D) in vitro models aim to closely mimic native microenvironments, and have emerged as an alternative to routinely used methods. In recent years, there has been an increasing interest in utilizing bio-printing and microfluidics technologies for 3-D tissue model generation. These approaches can provide 3-D heterogeneous structural and functional tissue models. Advances in bio-printing and microfluidic technologies have the potential to create new ways for 3-D tumor models generation for personalized medicine.

PLENARY SESSION 6 – NOT-FOR-PROFIT ORGANIZATIONS AND CONTRIBUTIONS TO ADVANCING CELL THERAPIES

SATURDAY, MAY 30TH, 2015 • TIME: 1530 – 1700
Roman Ballroom

Chair: C. Randal Mills

Academic and not-for-profit organizations play an essential role in the development of emerging cell therapy platforms which complements the efforts of industrial players in this space. With an emphasis on innovation, mechanism of action and nimble adjustments to manufacture and delivery – academia provides real time insights to the shared goal of wide scale deployment of effective cellular therapies. This plenary highlights such efforts by three internationally recognized not-for-profit consortiums: CIRM, Catapult and CCRM, and their model to impact the field of cell-based therapies.

THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE (CIRM)
C. Randal Mills

The California Institute for Regenerative Medicine (CIRM), was created by the people of California in 2004 to accelerate stem cell treatments to patients with unmet medical needs. To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast-track the development of today's most promising stem cell technologies.

With $3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

CIRM recently launched CIRM 2.0, a radical overhaul of the way we do business, that places emphasis on speed, partnerships, and patients. This presentation will review the implementation of this exciting new program and offer ways that CIRM might be able to help accelerate your stem cell technology.
CCRM – CANADA’S RM COMMERCIALIZATION VEHICLE: PROGRESS AND STATUS AFTER FOUR YEARS OF BRIDGING THE GAP

Michael May

The Centre for Commercialization of Regenerative Medicine (CCRM) has been in operation for 4 years as a sector-focused translation centre dedicated to generating value from the rich discovery pipeline of the Canadian regenerative medicine community. The CCRM was founded with investment from federal funding aimed at establishing the networks required to build a sustainable and viable ecosystem for commercialization. Since its inception CCRM has gained access to IP from leading academic institutions, established an industry consortium of 45 global companies, evaluated hundreds of technologies, conducted development projects using dedicated in-house facilities and bundled technologies into unique new company incubations. Dr. May will share lessons learned, describing how CCRM’s strategic plan is evolving to address key bottlenecks by leveraging past achievements and a growing network of stakeholders.

ACCELERATING THE GROWTH OF THE INDUSTRY BY SUPPORTING COMMERCIALIZATION

Keith Thompson

The cell therapy industry has enormous potential to grow health and wealth but has been characterized as risky and uncertain. The Cell Therapy Catapult has been established as a not for profit company to accelerate the development of the industry in the UK. The company has built facilities and teams of experts to support the growth of the industry through collaboration with industry and by forming spin outs to develop clinical assets and platform technologies. A diverse portfolio of projects has been established, ranging from TCR immune cells to tissue engineered organs, the progress with these projects and their contribution to lowering barriers to investment will be discussed.

Technical Session Summaries

TECHNICAL SESSION 1 – TECHNICAL ISSUES IN CORD BLOOD

THURSDAY, MAY 28TH, 2015 • TIME: 0800 – 0900
Florentine Ballroom I/II

Chair: Sue Armitage

Cord blood (CB) currently plays an important role in stem cell transplantation and cellular therapy, with an emerging role in regenerative medicine. Factors shown to influence the outcome of CB transplantation include the dosage of hematopoietic progenitor cells and total nucleated cells (TNC) and the degree of HLA matching. In development of quality inventories CB Banks need to consider the critical issues for a high quality collection, to meet all potential uses.

This technical workshop will discuss methods of CB collection and the critical elements for a high quality collection. The impact of who collects, the technique used to collect, the timing of the collection, where the collection is performed, and how collection personnel are trained and monitored are all aspects that affect the quality of CB collected, whether in-utero or ex-utero techniques are used. Improvement in quality can be achieved by identifying best current practices and encouraging new collection techniques.

CORD BLOOD COLLECTION 101
Robin Smith Berger

- Why collect cord blood
- Options for parents who want to save or donate cord blood
- Collection techniques and training

HOW CAN WE IMPROVE CORD BLOOD COLLECTION QUALITY?
Sue Armitage

- Discussion of the different collection models and comparison of volumes, total nucleated cell counts, viability and contamination rates
- Review of gestational age and weight of placenta on volume, total nucleated cell count and CD34+ cell count
- The effect of training techniques and the experience of the collector on the volume and contamination rate of the cord blood collected.

CORD BLOOD COLLECTION QUALITY: FUTURE CONSIDERATIONS
Karen Foster

- Over time the banking community continues to evolve using staff collectors performing ex utero collections to realizing the value of the remote collection model that uses remote clinicians performing in utero collections: indications for
use are also changing from being primarily hematopoietic reconstitution.
• Research and clinical studies with new indications will likely redefine the purity and potency of a usable unit, while significantly increasing the demand for cord blood collections.
• Such trends provide our cord blood community of both family and public bankers incentive to collaborate to establish best practices for achieving optimum collection quality from a broader range of collection channels.
• This includes collaboration to standardize training instructions and education programs, as well as creating a common voice to communicate to providers of consumables the innovations that will add value to cord blood banking.

TECHNICAL SESSION 2 – GETTING PERMISSION TO USE THE CLINIMACS CD34-SYSTEM: HOW TO CONVINCE YOUR IRB IT’S NOT RESEARCH

FRIDAY, MAY 29TH, 2015 • TIME: 0800 – 0900
Florentine Ballroom I/II

Chair: Carolyn Keever-Taylor

The CliniMACS CD34-System may be used in the United States under three main pathways: 1. As a Humanitarian Use Device (HUD) under the Humanitarian Device Exemption (HDE) regulations. 2. Through the single emergency use IND regulations, and 3. Under IND or IDE for new or ongoing clinical research studies. In each case there are requirements for approval by the local institutional review boards (IRBs). This session will include speakers who have obtained these approvals and who will explain when a given pathway should be used and how to obtain the needed approvals.

HUD APPROVAL AND APPROVAL FOR NON-RESEARCH USE
Carolyn Keever-Taylor

The CliniMACS CD34-Reagent System requires review and approval by the institutions IRB for use as an HUD. Documentation of this approval must be provided to Miltenyi before the CD34 Reagent System can be distributed. This applies whether or not the system is to be used for its approved indications. Separately or as part of the HUD approval, IRB approval to use the system for indications that are not approved but are not research is also required. This talk will describe what is required for IRB approval:
• As a HUD for HDE indications
• For non-research and non-approved indications

IRB REQUIREMENTS TO OBTAIN PERMISSION PRIOR TO THE PURCHASE OF THE CLINIMACS CD34-SYSTEM AS A HUD USAGE
Ann LeFever

• CliniMACS CD34 Reagent System Humanitarian Use Device approval and indications for use
• Materials required by Miltenyi prior to supplying the CliniMACS CD34 Reagent system
• Information to be submitted to the Institutional Review Board
• Is a specific Informed Consent document required for this usage?
• IRB requirements for submission of materials for each usage of the CliniMACS CD34 Reagent System

A SINGLE CENTER EXPERIENCE OBTAINING REGULATORY APPROVAL FOR USE OF THE CLINIMAX AFTER HUD APPROVAL
Joanne Kurtzberg

TECHNICAL SESSION 3 – TECHNICAL TOOLS OF IMMUNOTHERAPY: EXPANSION OF NON-ANCHORED CELLS

SATURDAY, MAY 30TH, 2015 • TIME: 0800 – 0900
Florentine Ballroom I/II

Chair: Paul Eldridge

Dr. Lapteva will discuss methods of NK cell expansion from peripheral blood using genetically-modified K562 cells expansion from NK cell lines. She will also address retroviral transduction and shipment of large numbers of NK cells for clinical use.

Dr. Rivière will describe the manufacturing platform of T-lymphocytes expressing specific chimeric antigen receptors (CARs) that presently supports multiple phase 1 clinical trials at MSKCC for the treatment of leukemia and lymphoma, as well as mesothelioma and prostate cancer. T cells genetically modified with a replication-defective gamma-retroviral vector encoding a CAR can be expanded upon selection and activation with CD3/CD28 magnetic beads. This bioprocess allows the generation of clinical doses in less than 2 weeks using the WaveTM Bioreactor. CAR T-cell characterization, release criteria and biological activity will be discussed.

Dr. Tonn will discuss the clinical scale isolation of antigen specific T-cells using the Streptamer technology including CMV and leukemia antigen (WT-1, PRAME) specific T-cells. First experience with the GMP compliant Expamer™ T-cell expansion system will be presented.
LARGE SCALE MANUFACTURING OF CLINICAL-GRADE NATURAL KILLER CELLS
Natalia Lapteva

- Methods of NK cell expansion
- Retroviral transduction of NK cells
- Shipment of large numbers of NK cells that improved NK cell expansion and persistence in patients

Presentation includes contributions from Natalia Lapteva, Robin Parihar, Juan Vera, Ann Leen, Lisa Rollins, Frits van Rhee, Hans Klingemann, Zhuyong Mei, Adrian P. Gee and Cliona Rooney

ENGINEERING CAR-T CELLS FOR CANCER IMMUNOTHERAPY
Isabelle Rivière

- Manufacturing platform for T lymphocytes expressing chimeric antigen receptors (CARs)
- CAR T-cell characterization, release criteria and biological activity
- Application in phase I clinical trials in subjects with leukemia, lymphoma, mesothelioma and prostate cancer

CLINICAL SCALE ISOLATION AND EXPANSION OF UNTouched ANTIGEN SPECIFIC T CELLS
Torsten Tonn

The presentation will address critical technical issues in the clinical scale isolation of antigen specific T cells using Streptamer™ technology. While CMV specific T cells will be the main focus (Odendahl M., et al. Cytotherapy. 2014;16(9):1245), first experiences in using a reversible GMP-compliant T cell expansion reagent (Expamer™) will be presented.

Workshop Summaries

WORKSHOP 1 – IMMUNOTHERAPY COMBINATIONS: JOINT SESSION WITH SITC

THURSDAY, MAY 28TH, 2015 • TIME: 1600 – 1730
Florentine Ballroom I/II

Co-Chairs: Bruce Levine and Hideho Okada

Recent publications have demonstrated remarkable successes of adoptive cell therapy for cancer, such as the use of tumor-infiltrating lymphocytes in patients with melanoma and autologous T lymphocytes genetically engineered to express chimeric antigen receptor (CAR) in patients with B cell malignancies. This is a joint session with Society for Immunotherapy of Cancer showcasing recent studies using multiple approaches to improve the efficacy of immunotherapies, especially in developing successful therapies for solid cancers. Dr. Zihai Li will discuss deletion of gp96 in regulatory T-cells (Tregs) to abrogate the suppressive function of Tregs, thereby improving the efficacy of adoptive T cell therapy of large melanoma. Dr. Muzaffar Qazilbash will present data from their phase II study evaluating idiotypic-KLH-primed, activated T cells in patients with multiple myeloma. Lastly, Dr. Hideho Okada will present a novel CAR therapy targeting glioblastoma-specific antigen EGFRviii. The session chairs encourage productive discussions with the audience.

TAMING TREGS FOR ADOPTIVE T CELL THERAPY OF CANCER
Zihai Li

- Tregs suppress anti-tumor effector T cells.
- gp96/grp94 is an essential chaperone for integrins and LRRC32 (GARP).
- Genetic deletion of gp96 in Tregs compromised their suppressive function.
- Ablation of gp96 in Tregs enhances adoptive T cell therapy of large melanoma.

IDIOTYPE-KLH PRIMED, ACTIVATED T CELLS FOR ADOPTIVE CELLULAR THERAPY OF MYELOMA
Muzaffar Qazilbash

- Idiotype (Id) is a myeloma-specific antigen
- Id/KLH-primed, CD3/CD28 activated T lymphocytes have potent anti-myeloma activity
- We conducted a randomized, phase 2 trial of Id/KLH vaccine + activated T lymphocytes in patients with multiple myeloma
- 38 of the planned 40 patients have been enrolled on the study
BRAIN TUMOR CELLULAR THERAPY
Hideho Okada

- My experience/study using alpha DC1 vaccine in patients with recurrent high-grade glioma (HGG)
- My collaboration with U Penn on CAR therapy – mostly published preclinical data
- Brief review on the recent progress of the field of brain tumor cellular immunotherapy.

WORKSHOP 2 – APPROACHES TO IMPROVE THE DEVELOPMENT OF CELL-BASED REGENERATIVE MEDICINE PRODUCTS VIA REGULATORY SCIENCE AND ADVOCACY

THURSDAY, MAY 28TH, 2015 • TIME: 1600 – 1730
Florentine Ballroom III/IV

Chair: Michael Mendicino

The FDA defines regulatory science as the science of developing new tools, standards, and approaches to assess the safety, efficacy, quality, and performance of all FDA-regulated products. For emerging technologies such as cell-based regenerative medicine products, numerous regulatory and technical challenges arise during product development. These challenges can include differences in donor and tissue sources as well as manufacturing diversity, potentially impacting product characteristics, or “critical quality attributes”, which are often not well-characterized. Other challenges include assay accuracy and reproducibility, which can be important for batch-to-batch consistency as well as product comparability. Finally, challenges arise for emerging technologies that require legislative action and/or stakeholder collaboration to improve and/or promote product development, approval and beyond. The speakers and panelists will review and discuss examples of approaches taken to utilize regulatory science and advocacy to help mitigate the challenges described, and contribute to the development of cell-based regenerative medicine products.

CELL-BASED REGENERATIVE MEDICINE PRODUCTS FOR CLINICAL TRIALS – REGULATORY TRENDS, CHALLENGES AND OPPORTUNITIES
Michael Mendicino

The approach for regulatory science to be discussed is analyzing trends so stakeholders can review lessons learned and focus on relevant challenges and opportunities. Trend analyses can come from:

- Publications and presentations from regulators
- Publications and surveys from professional organizations
- Mining of clinical trial databases
- Other sources

THE ALLIANCE FOR REGENERATIVE MEDICINE: REGULATORY STRATEGIES FOR REFORM
Michael Werner

- Provide an overview of the Alliance for Regenerative Medicine
- Describe ARM’s regulatory reform priorities
- Discuss specific regulatory ideas including: those included in 21st Century Cures and the National Center of Excellence for Regenerative Medicine Standards

REGULATORY SCIENCE FOR IMPROVED CHARACTERIZATION OF CELL-BASED PRODUCTS: CBER’S MSC CONSORTIUM
Steven Bauer

This talk will discuss some of the regulatory science challenges for cell-based products and describe how CBER’s MSC Consortium is addressing:

- Assessing predictive value of product characteristics
- Correlation of product characteristics with biological activities of cell-based products
- Development of product characteristics predictive of safety and effectiveness for cell-based products

THE ROLE OF MEASUREMENTS AND STANDARDS IN A REGULATED ENVIRONMENT
Anne Plant

- Good measurement process is essential for a successful product, not just for regulatory approval.
- Assuring confidence in measurements of complex biological systems is challenging; achieving robust protocols will probably require a community effort.
- Documentary standards often are enabled by demonstration of robust measurement protocols.

Panelist: Natalie Mount, PhD, Cell Therapy Catapult, UK

WORKSHOP 3 – STEM CELLS FOR NEUROLOGICAL DISORDERS

FRIDAY, MAY 29TH, 2015 • TIME: 1115 – 1230
Roman Ballroom

Chair: Josef Priller

This workshop will discuss current progress toward developing cell and gene therapies for central nervous system (CNS) disorders. Human mesenchymal stromal cells (MSCs) can be
used to secrete factors for tissue repair and disease correction in the brain. Jan Nolta will present the results of experimental studies, as well as the planned clinical trial to use MSCs to deliver brain-derived neurotrophic factor (BDNF) to rescue striatal neurons in Huntington’s disease. Chotima Böttcher will present a population of myeloid progenitors that target the CNS, and attenuate disease progression in animal models of amyotrophic lateral sclerosis and Alzheimer’s disease. The workshop will highlight future prospects and challenges of cell-based therapies in the CNS.

STEM CELLS FOR BRAIN REPAIR
Josef Priller

- Types of stem and progenitor cells used for brain repair
- Problems of cell and gene therapy in the CNS
- Examples of clinical trials in Neurology

MESENCHYMAL STEM/STROMAL CELLS ENGINEERED TO PRODUCE BRAIN-DERIVED NEUROTROPHIC FACTOR AS A POTENTIAL TREATMENT FOR HUNTINGTON’S DISEASE
Jan Nolta

- Discuss an overview of MSC-based cell and gene therapy
- Briefly discuss the therapeutic target - Huntington’s disease, where BDNF levels are blocked at the transcriptional level by a mutant protein
- Review the logistics of performing IND-enabling studies in an academic setting
- Summary of progress to date and introduce PRE-CELL, the observational lead-in clinical trial which is currently accruing

HEMATOPOIETIC PROGENITORS AS A TOOL TO TREAT NEURODEGENERATIVE DISORDERS?
Chotima Böttcher

- Bone marrow-derived cells for brain repair
- Novel cell population targeting the damage brain and providing therapeutic effects
- Perspectives for future translational studies

WORKSHOP 4 – CELL BASED ASSAYS AS POTENCY RELEASE CRITERIA FOR MSC: JOINT SESSION WITH IFATS

FRIDAY, MAY 29TH, 2015 • TIME: 1600 – 1730
Florentine Ballroom I/II

Chair: Luc Sensebé

The increasing use of MSCs, their definition as advanced-therapy medicinal products (ATMP) by European regulations, and the US Food and Drug requirements for their production and use imply the use of production processes that should be in accordance with Good Manufacturing Practices (GMPs).

One of the main concerns in the field is the lack of relevant controls and release criteria for safety and efficacy. Although regulatory authorities’ quality assurance metrics partially address safety issues in the manufacture of stem cell-based products, no standardized guidelines currently exist for the evaluation of stem cell functionality. The workshop will update the general vision of release criteria in clinical applications of MSCs. The 3 renowned speakers acting in MSC field and involved in clinical trials will share their vision of the potency release criteria. A special focus will be done on release criteria for immunological purposes.

MSC BASED THERAPY FOR SEVERE OSTEOARTHRITIS OF THE KNEE: THE ADIPOA EXPERIENCE
Christian Jorgensen

- Identify specific signature associated with MSC immunosupressive effects
- Potency assay associated with osteoarticular applications: where do we stand
- MSC for osteoarthritis. implication of IL1RA expression

IMMUNOLOGICAL ASSAYS AS POTENCY RELEASE CRITERIA FOR MESENCHYMAL STROMAL CELLS (MSCS)
Mauro Krampera

- Defining the main features of human MSC and immune effector cells for testing the immune regulatory potential
- Defining the mechanisms of action that better characterize human MSC immunological properties
- Defining robust markers and assays predictive of immunological potency
- Defining the best pre-clinical research in animal models of adoptive cell therapy that provide important insights on mechanisms of action

ADIPOSE BASED CELL ASSAYS
Jeffrey Gimble

- There are currently >160 adipose cell based studies registered with http://www.clinicaltrials.gov.
- An ISCT and IFATS consensus document has identified a series of assays for routine characterization of adipose-derived stromal/stem (ASC) and stromal vascular fraction (SVF) cells.
- The proliferation of non-bone marrow-derived MSC clinical studies has raised regulatory concerns regarding a reliance on immunophenotypic cell assays, prompting renewed emphasis on functional outcomes assays.

www.isct2015.com
WORKSHOP 5 – ADVANCES IN CELL THERAPIES AND REGENERATIVE MEDICINE IN PULMONARY DISEASES AND CRITICAL ILLNESSES

FRI DAY, MAY 29TH, 2015 • TIME: 1600 – 1730
Florentine Ballroom III/IV

Chair: Daniel J. Weiss

Progress in lung regenerative medicine in advancing at an increasingly rapid pace. This includes continued appreciation of endogenous lung progenitor cells that function in repair form injury as well as advances in techniques by which embryonic and induced pluripotent stem cells can be differentiated into lung airway and alveolar epithelial cells. In parallel, substantive progress has been made in understanding the mechanisms by which mesenchymal stromal cells and other adult stem and progenitor populations modulate lung inflammation and injury. This has led to a steadily increasing number of well-regulated clinical trials in lung diseases and clinical illnesses. Further, advances in ex vivo lung bioengineering continue to offer novel approaches for lung regeneration. The workshop features talks from three leading investigators focused on the types of cells that might be utilized in cell therapy approaches for lung diseases and critical illnesses, the recently published Phase 1 multicenter trial of MSCs for patients with the adult respiratory distress syndrome, and on recent advances in ex vivo lung bioengineering with decellularized whole lung scaffolds.

MESENCHYMAL STEM CELLS FOR THE TREATMENT OF ARDS: RESULTS OF A PHASE 1 CLINICAL TRIAL
Jennifer Wilson

- Review of rationale for use of MSCs in ARDS
- Phase 1 trial design
- Phase 1 results
- Phase 2 trial design

IT'S NOT JUST MSCS: OTHER ADULT STEM CELL POPULATIONS FOR USE IN CELL THERAPIES FOR LUNG DISEASES AND CRITICAL ILLNESSES
Daniel J. Weiss

- Review of current status of MSCs and EPCs
- Review current data with bone marrow-derived mononuclear cells
- Review current data with amniotic fluid-derived cells

LUNG BIOENGINEERING AND REGENERATION BASED ON NATIVE LUNG SCAFFOLDS
Sarah Gilpin

- Human and porcine lung decellularization methodologies
- Choice of cell sources for recellularization
- Biomimetic culture and functional assessment of human recellularized lung constructs

WORKSHOP 6 – IMMUNOMODULATION

SATURDAY, MAY 30TH, 2015 • TIME: 1345 – 1515
Florentine Ballroom I/II

Chair: Yufang Shi

In the recent years, animal studies and clinical investigations have demonstrated that mesenchymal stem cells are effective in treating various diseases with inflammatory abnormalities. This session will focus on the interaction between mesenchymal stem cells and immune responses and the role of this interaction in exerting therapeutic effects. It is anticipated that the presentations will provide information for better designing of protocols for clinical application of mesenchymal stem cells, especially for inflammatory disorders.

STEM CELLS IMMUNOLOGY AND PATHOLOGICAL AND THERAPEUTIC IMPLICATIONS
Yufang Shi

- Mesenchymal stem cells (MSCs) are specifically attracted to damaged tissue sites and are believed to closely interact with inflammatory factors and cells during tissue repair and regeneration.
- MSCs play a key role in regulating immune responses.
- The immune regulatory capacity of MSCs is plastic and varies according to the type and intensity of inflammation.
- Exogenously administered MSCs are believed to promoted tissue regeneration mainly through inflammation-induced cell empowerment

BRINGING MESENCHYMAL STEM CELLS INTO THE CLINIC
Katarina Le Blanc

- Mesenchymal Stromal Cells (MSCs) have immune-modulatory properites.
- MSCs have been infused intravenously, no acute infusional toxicity has been reported.
- Upon contact with blood, MSCs initiate activation of the complement and coagulation cascades.
MYELOID PRECURSORS AS TARGETS OF MESENCHYMAL STROMAL CELL THERAPEUTICS
Francesco Dazzi

- Mesenchymal stromal cells educate myeloid differentiation and function
- MSC induce the differentiation of myeloid progenitors into monocytes/macrophages
- These MSC educated myeloid cells exhibit immunosuppressive and regenerative properties

Advanced Practice Professionals Track Summaries

ADVANCED PRACTICE PROFESSIONALS TRACK 1 – ADVANCES IN CELLULAR THERAPIES

THURSDAY, MAY 28TH, 2015 • TIME: 1115 – 1230
Capri Room

Chair: Tobi Fisher

Cellular therapy has emerged as a rapidly growing field in oncology with various T and NK cell-based approaches. We will perform an overview of Novel T-cell therapies, as well as review the requirements for performing studies under GCP (Good Clinical Practice). New immunotherapeutic strategies are being developed as potential cancer therapies with the use of natural killer cells (NK cells), which are important mediators of tumor immunosurveillance and are active against certain hematologic malignancies. We will explore how cancer cells evade the NK-mediated immune surveillance. We will also focus on novel strategies to enhance NK cell function against cancer, including the use of genetically modified Cord blood-derived NK cells to enhance anti-tumor specificity.

NOVEL T CELL THERAPIES ON IND STUDIES
Helen Heslop

- Overview of novel cell therapies tested in clinical trials
- Requirements for performing studies under GCP
- Single patient INDs for cell therapy studies

APPLICATION OF NATURAL KILLER (NK) IMMUNOTHERAPY IN THE TREATMENT OF CANCER
Katy Rezvani

- Understanding the role of the immune system in the control of cancer and the mechanisms mediating immune evasion remains one of the most challenging questions in tumor immunology.
- Cancer cells evade NK-mediated immune surveillance by actively modulating NK cell function and phenotype, through the release of immunomodulatory molecules such as IL-10 and TGF-β1.
- Our group is exploring a number of avenues to enhance NK cell function against cancer.
- These include novel strategies to expand off-the-shelf cord blood (CB) derived NK and genetic modification of CB-derived NK cells to redirect their specificity

NOVEL T CELL THERAPIES
Bambi Grilley

This session will present data on Novel T Cell Therapies being conducted in the US under an investigator initiated IND and discuss:

- Types of studies in the clinic
- Preliminary clinical results
- Clinical trial operational issues
- How to handle patient specific treatments that may result from those therapies

ADVANCED PRACTICE PROFESSIONALS TRACK 2 – CHRONIC GVHD: REVISED SCORING, CASE PRESENTATIONS, ADVANCES IN TREATMENT

THURSDAY, MAY 28TH, 2015 • TIME: 1400 – 1530
Capri Room

Chair: Kristen Kindsvogel

CHRONIC GVHD: REVISED SCORING, CASE PRESENTATIONS, ADVANCES IN TREATMENT
Paul Carpenter

Chronic graft versus host disease will be reviewed, including physical exam, recently revised NIH scoring system, standard and more recent treatment approaches. Case studies will be presented for audience participation as well as to demonstrate the important roles of ancillary and supportive care.
ADVANCED PRACTICE PROFESSIONALS TRACK 3 – NERVOUS SYSTEM REPAIR/PULMONARY REGENERATION

THURSDAY, MAY 28TH, 2015 • TIME: 1600 – 1730
Capri Room

Chair: Alison Gulbis

Over the past few years tremendous progress has been made in the understanding of stem cell biology, devising sources of cells for transplantation, and cell delivery and targeting to affected areas in the body. Pulmonary regeneration and nervous system repair using cellular therapies are areas garnering great interest. Despite advances in pharmacologic approaches, there is still no cure for many diseases of the lung and they continue to cause significant morbidity and mortality. Though lung transplant is an option, it is limited by lack of donor lungs and complicated by the need for lifelong immunosuppression to prevent acute and chronic rejection. Through a better understanding of endogenous lung progenitor cell function, novel cellular therapy approaches have been developed to repair and regenerate the injured lung. We will gain a better understanding of endogenous lung progenitor cells, explore the novel cellular therapies for lung injury, and discuss lung tissue bioengineering.

Diseases of the nervous system affect millions of people worldwide. In the past 10 years, there has been increased interest in the possible uses and reparative properties of cellular therapies for neurologic disorders. We will review the current status of cellular therapies for nervous system repair.

ADVANCES IN LUNG REGENERATIVE MEDICINE
Daniel J. Weiss

- Current understanding of endogenous lung progenitor cells: how does the lung repair itself
- Update in cell therapy approaches for lung diseases
- Science fiction coming to life: growing lungs in the laboratory

GAME CHANGERS: USING CORD BLOOD TO HELP THE BRAIN
Joanne Kurtzberg

Cord Blood has been successfully utilized as a source of hematopoietic stem cells for rescue after myeloablative therapy for over 2 decades. Use without complete HLA matching is possible, likely due to immune tolerance of fetal and newborn lymphocytes. After observing that cord blood cells engraft in the brain after myeloablative allogeneic unrelated donor intravenous transplantation in pediatric patients with leukodystrophies, we hypothesized that microglial/oligodendrocyte cells could be manufactured from cord blood and used as an adjuvant therapy in genetic and acquired brain disorders. In addition, we identified paracrine activities of cord blood cells that we propose can exert trophic effects to reduce inflammation and influence brain repair after brain injury. In this session, I will present results of transplantation and infusion of allogeneic and autologous cord blood cells in patients with genetic diseases and acquired brain injury. Development, validation, characterization and design of a first in man clinical trial using DUOC-01 will also be described.

Quality and Operations Track Session Summaries

QUALITY AND OPERATIONS TRACK 1 – DESIGNING A GMP FACILITY

THURSDAY, MAY 28TH, 2015 • TIME: 0800 – 0900
Pompeian Ballroom III/IV

Chair: Shirley Bartido

Designing GMP (Good Manufacturing Practice) facilities for product scale-up and/or small-volume manufacturing is a complex, often expensive facility undertaking. GMP-compliant facilities are becoming increasingly common as more organizations want to improve patient safety even for innovative products and treatments that are still under development. Building even a small GMP facility involves meeting strict international regulatory standards, resolving numerous technical issues, and absorbing inevitably higher costs. Designing a large-scale GMP production facility for biological production requires various types of risk assessments to be carried out. This is the main tool in obtaining a balance between the aspects where GMP and biosafety guidelines contradict each other. Only by evaluating the various risks involved in the project, can rational and optimal choices be made regarding facility design and construction.
DESIGN AND COST OF AN ACADEMIC GMP FACILITY
Shirley Bartido

Discussion of the extensive pre-planning, cost analysis, and specialized consulting that are necessary to mitigate the many challenges in the following aspects:

- structural design
- critical mechanical & utility systems
- product and process flows and
- validation & regulatory issues

The perspective of the architect in the development and design of a cGMP facility will also be presented. As a member of the qualified design team, she will discuss the necessity of establishing the design team early on so that GMP requirements are comprehended and effectively incorporated in the initial planning stages of the project to ensure the innovative and economical design, construction, qualification, validation and operation of the facility.

DESIGNING A CGMP FACILITY: PERSPECTIVE OF THE ARCHITECT IN THE DEVELOPMENT AND DESIGN OF A CGMP FACILITY
Carylynn Simmons

A cGMP facility is more complex and costly than most buildings as they are highly regulated and must be in strict compliance with the current Good Manufacturing Practices as set out by the FDA. A qualified design team comprehends and effectively incorporates GMP requirements from the very beginning of a project ensuring the innovative and economical design, construction, qualification, validation and operation of the facility. Methodologies of experienced design professionals will be discussed as they contribute to the successful delivery of a cGMP facility:

- Vetting highly skilled and collaborative team members
- Early identification of project goals and issues
- Define manufacturing processes
- Timely selection of critical systems
- Establish meetings to promote attendance of key decision makers
- Include contractor and consultants early in the design process
- Engage Facilities Maintenance and Operations and Information Technology staff early
- Prioritize requirements and materials as they relate to operation in order to maintain schedule and budget

QUALITY AND OPERATIONS TRACK 2 – MAKING THE MOST OF AUDITS AND ENSURING DATABASE ACCURACY

THURSDAY, MAY 28TH, 2015 • TIME: 1400 – 1530
Pompeian Ballroom III/IV

Co-Chairs: Michele Sugrue and Deborah Lamontagne

Hematopoietic Progenitor Cell (HPC) transplant programs have a unique set of challenges to provide patients with consistent, high-quality care and cellular products that ensure positive outcomes. Establishing and performing an effective audit program, while time-intensive, is a critical element of quality improvement initiatives. This session will address approaches to determining and instituting audit key measures, acceptance criteria, and corrective actions. Examples of audit plans and reports for will be presented in order to demonstrate methods to obtain valuable and practical data. Speakers will describe best practices to meet accreditation standards and methods to avoid citations using case scenarios. The CIBMTR, which facilitates critical research and has led to increased survival and enriched transplant recipients’ quality of life, is a vital component of quality assurance efforts. Processes to collect and maintain reliable CIBMTR data will be addressed including targeted data review and compliance with the CIBMTR comprehensive audit program.

DEVELOPING AUDITS PROGRAMS AND REPORTS
Michele Sugrue

- Identify approaches to developing a meaningful audit program data
- Describe methods to determine key indicators and acceptance criteria
- Discuss processes to implement audit schedules and prepare audit reports

SUCCESSFUL AUDIT STRATEGIES TO MEET ACCREDITATION MANDATES AND AVOID CITATION
Pamela Jacobson

- Describe audits and outcomes of Corrective and Preventative Action (CA/PA) Modules
- Discuss assessments of the effect of change in determining success
- Examine audits of the performance of Standard Operating Procedures (SOPs)
- Describe approaches to audit outside agencies contracted to perform critical services
- Case Study – Use of Audit Data to Promote a “Safety Culture”
ENSURING DATABASE ACCURACY: CIBMTR’S AUDIT AND MONITORING PROGRAM
Debra Christianson

- Identify methods of ensuring data quality prior to audit, including: providing educational resources, revision of forms, forms instruction manuals, data field validation, and targeted review of data
- Describe CIBMTR audit and corrective action processes
- Describe CIBMTR clinical trial monitoring procedures to ensure effective review of research data
- Case Study: following a data field through the data quality processes

QUALITY AND OPERATIONS TRACK 3 – COMMISSIONING AND VALIDATION OF A GMP FACILITY

THURSDAY, MAY 28TH, 2015 • TIME: 1600 – 1730
Pompeian Ballroom III/IV

Chair: Shirley Bartido

Investigational products intended for human subjects’ research studies, including cell therapy products, are developed and manufactured within an institutional-standard Manufacturing Quality System (MQS). The MQS is designed to ensure compliance with regulatory requirements for products used in clinical trials. To satisfy these requirements, critical facility systems and equipment need to be validated with the appropriate organizations or groups responsible for the validation work.

Validation is defined as ‘establishing documented evidence which proves with a high degree of assurance that a specific process will consistently produce a product meeting its predetermined specification and quality attributes’.

CLINICAL MANUFACTURING FACILITY VALIDATION: “KEEPING IT SIMPLE”
Mark Shannon

Several thoughts and experiences will be shared that will hopefully help you become a better team leader or participant in the validation of your clinical manufacturing facility. The main areas covered in this presentation shall include:

- Budgets, Timelines and Project Management
- Keeping your scope aligned to hit the target
- Regulatory agency “buy-in” and participation
- Common pitfalls to avoid (and some sad stories)

QUALITY AND OPERATIONS TRACKS 4, 5, 6 – VALIDATION BOOT CAMP

Qualification, verification, validation. These terms can cause confusion and disagreement in the cell therapy community. Some purists insist that the terms be used correctly, consistently and globally. Others are of the mindset that as long as you do something and define and use terms for your local practices, you are meeting the intent of accreditation standards and regulatory authorities. Aside from the terminology issues, the broad topic of “validation” utilizes significant resources and has multiple challenges for cell therapy facilities. The planning committee identified so many topics related to validation that we decided to present them together in a half day “Boot Camp” format. Each session will provide ample time for discussion, and two interactive scenarios will be discussed with a panel of experts comprised of all of the Boot Camp chairs and speakers and a representative of a smaller processing facility. Given the variety of topics and the expertise of speakers, participants in Validation Boot Camp are sure to learn some important tips they can use for future validations.

Panel Members: Renee Smilee, Lizette Caballero, Lynn O’Donnell, Emily Hopewell, Ronit Slotky, Carolyn Keever-Taylor, Rosemarie Bell and Deborah Lamontagne

QUALITY AND OPERATIONS TRACK 4 - VALIDATION BOOT CAMP PART I: CONCEPTS OF VALIDATION AND QUALIFICATION

FRIDAY, MAY 29TH, 2015 • TIME: 0800 – 0900
Pompeian Ballroom III/IV

Chair: Renee Smilee

This first session will cover the basic concepts, terminology and challenges such as starting material sourcing and data analysis.

QUALIFICATION, VALIDATION AND VERIFICATION: SAY IT AGAIN?
Lizette Caballero

- Define the concepts of: Validation, Verification and Qualification
- Discuss what is the difference and similarities between the three concepts
- Share validation, qualification and verification examples
- Give a short statistics course in how to interpret/analyze your data
QUALITY AND OPERATIONS TRACK 5 – VALIDATION BOOT CAMP PART II: EQUIPMENT AND SUPPLIES

FRIDAY, MAY 29TH, 2015 • TIME: 0915 – 1045
Pompeian Ballroom III/IV

Chair: Lynn O’Donnell

This second session will cover validation – or verification or qualification as the case may be – of equipment, supplies and entire facilities, with two speakers presenting their own experience with a recent validation and third interactive session on moving a laboratory.

VALIDATION OF AN X-RAY IRRADIATOR TO REPLACE A CESIUM IRRADIATOR
Emily Hopewell

This presentation will focus on the following points:
- Why do we irradiate cells?
- Why switch from a cesium source to an X-ray source?
- How do you demonstrate comparability between the two radiation sources?

THE DEXTRAN SHORTAGE – HOW TO APPROACH THE VALIDATION OF A NEW CRITICAL REAGENT
Ronit Slotky

This presentation will
- Describe the challenges that processing laboratories face when a critical reagent is no longer available, as uncovered by the recent Dextran shortage.
- Discuss the important considerations when assessing alternative reagents.
- Present one institution’s approach for handling the Dextran shortage.

MOVING YOUR FACILITY – QUALIFICATION, VERIFICATION, VALIDATION? ALL OF THE ABOVE (AND THEN SOME)
Panel Members and Audience

This interactive scenario will cover the following:
- Planning, preparing and execution of a validation plan
- Logistics, resources and reality of moving
- Hits and misses of planning

QUALITY AND OPERATIONS TRACK 6 – VALIDATION BOOT CAMP PART III: PROCESSING AND TESTING METHODS

FRIDAY, MAY 29TH, 2015 • TIME: 1115 – 1230
Pompeian Ballroom III/IV

Chair: Emily Hopewell

This third session will delve into process validations with multiple examples as well as provide a way to accomplish more with those expensive validation materials.

VALIDATION OF PRODUCT VOLUME TO WEIGHT CONVERSION, DUMP FREEZE CONDITIONS, AND METHODS FOR POST THAW FLOW CYTOMETRY
Carolyne Keeever-Taylor

The design, execution, and results of three processing or test methods will be presented for discussion. These include validation of:
- The conversion factor to equate HPC(A) product weight to volume
- Required conditions for product freezing at -80°C without temperature control (dump freeze)
- Conditions for the accurate enumeration by flow cytometry of cell populations in thawed samples

MANAGING MULTIPLE VALIDATIONS WITH LIMITED PRODUCT AVAILABILITY
Rosemarie Bell

This presentation will cover how to
- Plan multiple validation runs with limited product availability
- Assess multiple validation runs from an overall perspective
- Get the most out of sampling plans
- Organize data gathering for efficient reporting

VALIDATION OF NEW OR IMPROVED PROCESSING METHODS FOR BONE MARROW: RBC REDUCTION AND OTHER SCENARIOS
Panel Members and Audience

This interactive scenario will cover the following:
- Validation of the Sepax SmartWash protocol
- Achieving consistency in the face of staff turnover, starting product variation and different goals for adult and pediatric transplant programs
- Sourcing marrow or other starting material for validations
- Discussion of clinical utility vs. technical futility – when is a process good enough?
ENSURING QUALITY IN STEM CELL-BASED PRODUCTS FOR MULTI-CENTRIC CLINICAL TRIALS
Viraf Vasania

Moving a promising cell-based product from the early development phase through to clinical trials poses a number of challenges. Challenges include consistency of every batch, efficacy of cellular products and the combinatorial products, handling of cells on scaffolds, transport of cell-based products to various clinical trial centers and appropriate handling of cells by the clinicians. In this talk, we share our experiences on these aspects as we developed Limbal grafts, Conjunctival grafts and other cell combinations on scaffolds, at Reliance Life Sciences, for clinical studies. Stringent quality control checks at various steps of cell processing, transport and traceability supported by in-house enterprise software was very useful. Last but not the least, we bring you our efforts and strategies to ensure the appropriate handling of these grafts at the clinical trial site which was of paramount importance to us.

- Standardization of processes for preparation of cell-based products.
- Traceability of each cell-based product and raw materials used in the manufacturing process.
- Methods to ensure quality and consistency in the developed cell-based product.
- Transporting cell-based products to hospital sites across the country.
- Ensuring the correct usage of the cell-based products at the transplant site.

STEM CELL MEDICINE – ITS POTENTIAL AND HOW TO ENSURE QUALITY IN MULTIPLE TRIALS ACROSS DIFFERENT GEOGRAPHIES
Stephen Minger

This talk will:

- Investigate the global market for stem cell and regenerative treatments, looking at macroeconomic trends
- Explore the current regulatory environment of clinical trials and the need for standardization of quality control and human factors across sites (ACRES and GERI initiative)
- Explore these aspects in the context of manufacturing, as well as operational aspects such as cryopreservation and transportation.

PRODUCT RELEASE TESTING CONSIDERATIONS FOR DIRECTED ALLOGENEIC OR AUTOLOGOUS PRODUCTS
Sowmya Viswanathan

Using examples from clinical trials for immunotherapy (with a permanent Natural Killer cell line) and regenerative medicine (cardiac (endothelial progenitor cells (EPCs)) and osteoarthritis...
(mesenchymal stromal cells (MSCs)) applications, this talk will illustrate:

• the quality requirements in Canada for donor screening, sourcing of ancillary reagents and product release testing for directed allogeneic and autologous cell therapy products
• and will include discussions on associated costs for meeting these quality requirements, and how this in turn may affect reimbursement paradigms

CLINICAL TRIALS: MOVING CELL BASED THERAPIES TO THE CLINIC
Aisha Khan

The promise of cell-based therapies is evident in discovery research. The complexity that gives cells such promise, however, has led to regulatory concerns about safety and efficacy. This presentation will describe the need of quality standardization for an investigational new drug application. Presentation will be focused on the current regulatory and policy environment for developing human cell therapies:

• The role of clinical trials for the development of new therapies
• How ethical concerns are addressed in the oversight of clinical trials and consider emerging issues specific to stem-cell-based therapies
• How institutions involved in clinical trials address regulatory policy issues
• Lessons learned from IND/Phase I trials including overcoming the challenges, and addressing the regulatory issues, as stem cell based therapies move towards the clinic
• Considerations for early Phase Clinical Trial Design in Cellular Therapies
• Overcoming challenges to initiating cell therapy clinical trials in rapidly developing countries: India as a Model

QUALITY AND OPERATIONS TRACK 8 – RATIONALE AND CHALLENGES OF BUILDING A GMP FACILITY IN ACADEMIA: A ROUND TABLE DISCUSSION
FRIDAY, MAY 29TH, 2015 • TIME: 1600 – 1730
Pompeian Ballroom III/IV

Chair: Shirley Bartido

The session will include brief ten minute presentations by Keith Thompson of Catapult, Adrian Gee of Baylor College of Medicine and Isabelle Rivière of Memorial Sloan Kettering Cancer Center. This will include a historical perspective of their present facility and operations. This will be followed by a moderated round table discussion which would focus on the rationale and challenges that are related to academic facilities.

QUALITY AND OPERATIONS TRACK 9 – THE GOLDEN THREAD OF QUALITY
SATURDAY, MAY 30TH, 2015 • TIME: 0915 – 1045
Pompeian Ballroom III/IV

Co-Chairs: Kara Wacker and Alex Slobodianski

On the occasion of the recent release of the FACT Common Standards for Cellular Therapies (1st Edition), we invite attendees to join a discussion of quality as the backbone for any approach to provide robust, reliable, safe and effective cell-based therapies. As there are many different approaches, our basic view on quality may differ in many parts of the world, as does our view of the dynamics that quality should show in process development, preclinical testing and clinical trials. Concepts of quality are challenged in ATMPs and “351” cell products, and again, US and European views may differ but still have the same goal in mind: the safety of the recipient. We hope to ignite a vivid exchange of thoughts by touching upon issues related to regulatory expectations for quality and whether quality is a thread or barrier between early clinical research and translational medicine.

QUALITY ASPECTS OF ATMP DEVELOPMENT IN EUROPE
Alex Slobodianski

• Classification, regulatory tools and product specific guidelines of ATMPs in the European Community.
• Implementation of the ATMP Regulations within the European Union, pre-authorisation requirements and post-authorization requirements.
• Overview of European approaches to achieve the gold quality standards in the development of ATMPs.

REGULATORY EXPECTATIONS FOR QUALITY MANAGEMENT IN THE U.S.
Speaker: Bangon (Day) Longsomboon

• Describe the United States FDA’s expectations for quality management during clinical development of cellular therapies.
• Explain the difference between requirements for early clinical trials and for pre-market approval.
• Discuss the challenges of applying quality management principles after early clinical trials.
**ACADEMIC PROGRAM**

**USING THE FACT COMMON STANDARDS AS A STEPPING STONE**
*Phyllis Warkentin*

- Describe the purpose and scope of the FACT Common Standards.
- Discuss how the FACT Common Standards can help advance research.
- Explain how the FACT Common Standards for Cellular Therapies can bridge early clinical trials with GMP requirements.

**Audience Discussion**
*Moderator: Kara Wacker; Commentary from presenters*

- Answer questions from the audience.
- Invite the audience to discuss how quality may serve as a thread or an obstacle between early clinical trials and regulatory approval.

**QUALITY AND OPERATIONS TRACK 10 – TECHNOLOGY TRANSFER: ACADEMIA TO INDUSTRY (AND BACK AGAIN)**

**SATURDAY, MAY 30TH, 2015 • TIME: 1115 – 1215**
*Pompeian Ballroom III/IV*

**Chair: Mark Lowdell**

This one hour session is focused on the challenges associated with the transfer of product manufacture between industry and academia. The perceived “usual” route for drug development is from academia into industry. However, with patient-specific cell and tissue therapies becoming established as the leaders in commercialization at present, the need for hospital or academic based GMP capacity is very apparent both for the procurement of the starting material and often for the final finishing and fill of the trial product. Moreover, many small commercial cell therapy developers lack GMP capacity and seek contracts with professionally operated academic labs as small CMOs. The first speaker will be Moya Daniels who will discuss how to integrate a commercial product into an academic lab and how to manage the expectations of the commercial partner. The presentation will be followed by a 5 min Q&A session with both speakers.

The second speaker will present the challenges associated with the more traditional situation of academic manufacture to CMO. Most early phase cell and tissue therapy trials in the EU are still within academia or early spin outs and their first experience of commercial manufacture is when they are faced with transfer to the US. This talk will present the history of establishing CMO manufacture of a master cell bank with CMO 1 for a patient-specific therapy and WCB at CMO 2 and the subsequent scale-up of the working cell bank at CMO 3.

The session will conclude with a second 5 min QA session with both speakers.

**TECHNOLOGICAL DEVELOPMENT OF A CELLULAR THERAPEUTIC: INDUSTRY TO ACADEMIA AND BACK AGAIN**
*Moya Daniels*

I will present an overview of the elements to be considered when transferring a manufacturing process and testing of a cell therapy product to an academic institution in support of the clinical development of an industry sponsored technology. The following areas of focus will be addressed in the presentation.

- Operations
- Quality
- Regulatory

**MOVING A TRIAL FROM ACADEMIC GMP TO CMO CGMP – A REAL WORLD EXPERIENCE IN CROSSING THE ATLANTIC**
*Mark Lowdell*

This presentation will present the history of a real-life technology transfer of an adoptive cell therapy from a single UK academic EU GMP site into three US cGMP CMOs. It will focus on the differences between EU GMP and cGMP and the different expectations and limitations of academic versus CMO GMP with emphasis on

- Staff training and experience
- Paperwork
- Comparability of process and product

**QUALITY AND OPERATIONS TRACK 11 – STAFFING MODELS AND SUCCESSION PLANNING**

**SATURDAY, MAY 30TH, 2015 • TIME: 1345 – 1515**
*Pompeian Ballroom III/IV*

**Chair: Karl Stasko**

How do we in the cell therapy community navigate and respond to the growth and challenges posed by clinical, experimental, and regulatory needs? We do it together. Institutions rise and fall on the talents of their people. Budgets may dictate the limits of our resources, but with proper planning and strategy, we can make the most of a reality where quality over quantity counts. This session will provide a baseline look at the current state of staffing across the field. From this starting point, tactics will be provided to proactively advocate for growth in the present, and plan for the succession of talent in the future.
CHALLENGES IN PREPARING CELLULAR THERAPY LABS AND STAFFING FOR AN EVER-CHANGING FUTURE
Sarah Nikiforow

- The ever-changing demands on academic cellular therapy laboratories to keep pace with standard clinical and experimental product needs; to respond to medical, financial, and infrastructure initiatives dictated by the supporting institution; as well as to assimilate new technologies create challenges for the physical plant and staffing models.
- To respond appropriately requires an understanding of both upstream and downstream resources and constraints, the ability to predict future impact of current initiatives, and metrics by which to equate production volume with staffing needs.
- This presentation will address the experience of Dana-Farber Cancer Institute’s Cell Manipulation Core Facility in navigating these challenges and contrast it with models pursued by other major academic processing facilities.

STAFFING MODELS AND SUCCESSION PLANNING FOR THE CELL THERAPY LABORATORY
Suzanne Dworsky

Staffing Models:
- What are the regulatory requirements specifically as it relates to segregation of duties?
- What is the approach to staffing models, LEAN versus as many people as administration will let us have?
- What is affordable versus what is desired? How are staffing models related to productivity?
- What levels of staff are needed for the model to include discussion of required degrees, certifications, experience, and training? Is the requirement different for GTP versus GMP?

Succession Planning:
- What are the internal and external factors affecting staffing in the cell therapy laboratory?
- What does the future of laboratory staffing look like?
- What are the training models to support sustainability?
- What level of education is required to work in the cell therapy laboratory?
- Who gets trained for your job?
- How do you decide?

RESULTS FROM THE ISCT LABORATORY PRACTICES COMMITTEE CELL THERAPY LABORATORY STAFFING SURVEY
Joseph Mierski

In an effort to better understand how cellular therapy laboratories were being staffed the ISCT Laboratory Practices Committee (LPC) conducted an online survey. The survey had 3 goals including:
- Determine the current staffing levels in cell processing laboratories
- Identify potential staffing level benchmarks
- Determine the interest level for a certification of cellular processing scientists.

Results of the survey will be presented.

QUALITY AND OPERATIONS TRACK 12 – TECHNICAL TOWN HALL MEETING: DISCUSSION OF COMMON OR EMERGING ISSUES FACING CELL THERAPY FACILITIES

Chair: Vicki Antonenas

This session will be an interactive and open discussion that has been specifically arranged to invite all ISCT attendees to come along with their questions or concerns to be answered or be given guided information regarding common technical questions or emerging issues facing cell therapy facilities. Common topics such as current processing techniques versus new techniques, interpretation of test results, mishaps and close misses, producing constant products for clinical infusions, integration of new cellular therapy trials with the routine cell processing labs, databases, how best to document DLI efficacy, reducing transcription errors and human errors. A presentation will also be given by an independent lab expert regarding the failed engraftment incidents (and deaths) that occurred from a paediatric BMT unit in UK and lessons learned would be applicable to every laboratory cryopreserving cells.

The primary purpose of the session is to provide information to the attendees and the cell therapy facilities, and hear how others have dealt with the problem with input from a panel of expert persons and from the audience.

Panelists:
- Paul Eldridge – St. Jude Children’s Research Hospital, US
- Mike Watts – Wolfson Cellular Therapy Unit, University College London Hospitals, UK
- Heather Daley – Dana Farber Cancer Institute, US
- Renee Smilee – Moffitt Cancer Center, Cell Therapy Facility, US
Strategies for Commercialization Track
Session Summaries

STRATEGIES FOR COMMERCIALIZATION TRACK
1 – HOT TOPICS IN PROCESS AND PRODUCT
DEVELOPMENT: PARTICULATES, SERUM-FREE
MEDIA, AND BIOREACTORS

THURSDAY, MAY 28TH, 2015 • TIME: 0730 – 0900
Pompeian Ballroom I/II

Chair: Ohad Karnieli

As cell therapy evolves and matures towards late stage clinical
trials and marketing the need for process development
and upscaling becomes critical. The Process and Product
Development committee (PPD) of the ISCT survived and
ranked the main common issues and challenges of developers
and initiated a series of peer discussions and white papers
aiming to discuss and assist by peer knowledge in maturing the
industry. The lead topics are:

- Challenges and possible solutions for adaption of bioreactor
to cell manufacturing,
- Serum free media development
- Particulates in single use technologies.

The session will consist of three panel discussions on the
above with a wide variety of expert panelists in the subjects
discussed. The experts consist of cell therapy developers and
manufacturers, processing technology developers and vendors
of cell therapy product complimented by quality and regulatory
experts. The audience is encouraged to take an active part in the
discussions and ask questions.

Panelists:

Bioreactor:
- Harvey Brandwein, Pall Corporation, US
- Shannon Eaker, GE Healthcare, US
- Sarah Callens, Cell Therapy Catapult, UK
- Thomas Brieva, Celgene Cellular Therapy, US
- Eytan Abraham, Lonza, US

Serum Free:
- David Fiorentini, Biological Industries, IL
- Jessie Ni, Irvine Scientific, US
- Andrew Campbell, Thermo Fisher Scientific, Inc., US
- Christopher Bravery, Consulting on Advanced Biologicals Ltd, UK
- Steve Oh, Bioprocessing Technology Institute, SG
- Dominic Clarke, Charter Medical, US
- Jerry Martin, Pall Corporation, US
- Vinod Vilivalam, West Pharmaceutical Services, US
- Elizabeth J. Read, EJ Read Consulting LLC, US

STRATEGIES FOR COMMERCIALIZATION TRACK
2 – ANCILLARY MATERIALS

THURSDAY, MAY 28TH, 2015 • TIME: 1115 – 1230
Pompeian Ballroom I/II

Co-Chairs: Claudia Zylberberg and Lynn Csontos

The hot topics that we hope to cover in the Ancillary Materials
session at ISCT are as follows:

- Discussions of the ever changing global regulatory
  landscape for ancillary materials used for cellular therapy
  applications.
- Who is accountable for the qualification of ancillary
  materials?
- What does a risked-based qualification plan of an ancillary
  material include?
- Who pays for quality?
- What is the impact of quality on the cost of goods?
- When is the right time to switch from Research Grade to
  more compliant materials?
- What are the differences between in-process ancillary
  materials and final formulation raw materials?

Perspectives on all of these topics will range from academic,
translational and clinical research (from biotech and pharma).

Panelists:
- Jiwen Zhang, GE Healthcare
- Joanne Kurtzberg, MD, Duke University Medical Center
- Brian Newsom, Thermo Fisher Scientific Inc.
- Jennifer Solomon, PhD, STEMCELL Technologies Inc.
STRATEGIES FOR COMMERCIALIZATION TRACK 3 – POTENCY AND SAFETY ASSAY DEVELOPMENT

THURSDAY, MAY 28TH, 2015 • TIME: 1600 – 1730
Pompeian Ballroom I/II

Chair: Christopher Bravery

This session focuses on the difficulties and possible pitfalls when developing reliable assays to address safety and potency of cell therapy products. Assays critical to safety need special attention to ensure they are sufficiently sensitive, accurate and precise before clinical development starts. Potency assays are also essential but pose a range of issues for cell products whose mechanism/s of action can be diverse and difficult to elucidate. It’s important to develop a strategy to undertake biological characterisation around the mechanism of action to ensure by the end of development you understand the likely mechanism/s through which the product works. The objectives of this strategy should be not just to have one or more routine potency assays (potency matrix) but also other methods that measure biological activities to support manufacturing changes (comparability). Ultimately commercial considerations also play their part and refining analytical methods to ensure they are robust and can deliver a result in a timely manner without unduly impacting the cost of goods needs also to be factored-in.

QUALITY AND REGULATORY CONSIDERATIONS WHEN DEVELOPING POTENCY ASSAYS FOR LATE STAGE CELL THERAPY PRODUCTS

Robert Perry

- Potency Assays and the materials used in the assay must be able to be qualified and validated
- The assays need to be consistent and reproducible
- Mode of Action for the cell type will help to define biological relevance of the assay
- Potency assay is critical for the final design of your manufacturing process

CRITICAL CONSIDERATIONS FOR THE DEVELOPMENT OF CELL THERAPY POTENCY ASSAYS. CASE STUDY: ANGIOGENESIS

Claudia Zylberberg

- Characterization of therapeutic substances for cell therapy must meet desired biological function requirements.
- Potency assay development must take into account multiple factors: biological activity and nature of the products, such as cell source and culture method, function, in vivo cellular environment and administration method. Correlated to a clinical function.
- Potency assays must provide traceability, meet specifications, include reference materials, be quantitative, reproducible, properly controlled and appropriately documented. Multifactorial and Measurement of complex signals. Multiple MOA
- Standardization what can be done, initiatives

ASSAYS FOR DEVELOPING SCALABLE, COST EFFECTIVE HMSC MANUFACTURING PROCESSES

Jon Rowley

- Strategic Process Development is a key component for developing any commercially successful and competitive cell-based therapeutic.
- Well controlled assays for cell-based products and manufacturing intermediates are required for developing scalable and cost effective cell-based therapeutics.
- Data-driven decision making is critical for running efficient and effective process scale-up or streamlining campaigns
- Assays for identity, potency, purity and safety will be discussed.

STRATEGIES FOR COMMERCIALIZATION TRACK 4 – REIMBURSEMENT

FRIDAY, MAY 29TH, 2015 • TIME: 0800 – 0900
Pompeian Ballroom I/II

Chair: Richard Maziarz

The ISCT education session on reimbursement will address issues about reimbursement barriers that might face the cell therapy industry for new product development. Insights will be provided regarding the nature of payer support for novel cellular therapies within the United States as well as several countries in Europe. The session will close with a view from the corporate world and how one might successfully develop products, recognizing these national variations in reimbursement strategies and regulatory environments.

REIMBURSEMENT CHALLENGES AND OPPORTUNITIES IN HEMATOPOIETIC CELL TRANSPLANT: A CASE STUDY

Stephanie Farnia

- This session will share lessons learned about challenges and opportunities in healthcare reimbursement models through the experience of hematopoietic cell transplant (HCT).
- Discussion will include the impact of the Affordable Care Act on the reimbursement environment, issues unique to governmental and commercial payers
- Strategies will be presented on how to engage and partner with payers.
REGIONAL VARIATIONS IN CELL THERAPY REIMBURSEMENT ACROSS THE BIG5EU
Panos Kefalas

- Variation in pricing and reimbursement frameworks for cell-therapies across Big5EU (France, Germany, Italy, Spain and UK)
- Impact of therapy features including incremental benefit, size of target population and regulatory status on health technology assessment

DESIGNING CELL THERAPY TRIALS FOR REGULATORY AND HTA APPROVAL
Dawn Driscoll

Cell therapy trials should be designed in order to meet Regulatory and health technology assessment (HTA) bodies’ requirements. Ideally multiple regions’ requirements can be met within one well designed study. This talk will:

- Present basic concepts in trial design for international commercialization, and
- Present two case studies focused on reimbursement

GOOD COLD CHAIN DISTRIBUTION PRACTICE CONSIDERATIONS FOR PLANNING AND DESIGNING COST-EFFECTIVE COLD-CHAIN STRATEGIES
Kevin O’Donnell

- Hard lessons learned from the pharmaceutical industry
- Cost, stability and regulatory considerations

IMPACT OF COLD CHAIN DECISIONS ON CRITICAL QUALITY ATTRIBUTES AND COST OF GOODS
Brian Murphy

- Key considerations of product suitability for specific cryo/cold chain distribution models
- Engineering examples and lessons learned from Celgene Cell Therapy products experience

LOGISTIC REQUIREMENTS FOR CELL THERAPY PRODUCTS AND HOW THESE IMPACT COST
Michael Trocchia

- Four Ts of Cell Therapy Cold Chain Logistics
- Temperature Requirement; Ambient/Dry Ice/LN2
- Time in Transit/Expiry
- Transportation Provider; “White Glove”/Overnight Carriers
- Tracking; Active versus Passive

THE DECLARATION OF INVESTMENT: NOT ALL CELL THERAPIES ARE CREATED EQUAL
Reni Benjamin

- Introduction to the world of investment and finance
- What’s hot and what’s not

2014 was banner year for the cell therapy space. Sales of approved cell therapy products combined cleared the >$1B threshold and more products advanced through various stages of clinical development. In addition, significant capital was raised from marquee healthcare investors as a resurgence of the field took place. With all the excitement occurring in the background, the public equity markets remain very selective when considering investment opportunities. While clinical and regulatory challenges remain, we view the advancement of the cell therapy space to occur as biotechnology companies continue to take large risks, and clinical data and regulatory approvals help guide investor dollars to the next generation of therapeutics revolutionizing the healthcare industry. Our panel will focus on what’s hot and not in cell therapy and provide insight from several Wall Street analysts as to how investors view this exciting and novel therapeutic approach.
INVESTOR’S VIEWS OF THE EMERGING GENOMERX REVOLUTION
Joshua Schimmer

- Highlight emerging investor interest in gene therapy, gene editing and genetically engineered T cells
- Discuss opportunities and challenges for the field that investors are currently evaluating.
- Identify outstanding unmet needs which investors may find attractive

PARADIGMS IN HEALTHCARE – COULD THE “STEM CELL SPACE” BE NEXT?
Jason Kolbert

- We will examine the basics of Biotechnology Investing
- We will evaluate historical Paradigm shifts, such as monoclonal antibodies
- We will establish a framework to sort Cell therapy
- We will evaluate SWOT (strength – weakness – opportunities & threats) among Cell Therapy companies
- We will explore where is Big Pharma in the Cell Therapy Space

CCRM’S EVALUATION OF EARLY STAGE TECHNOLOGIES
Michael May

- Description of CCRM commercialization model
- Challenges and opportunities at the interface between academia and industry
- Establishing and nurturing critical collaborations and partnerships

ENVISIONING AN INDUSTRIAL CELL THERAPY MANUFACTURING ECOSYSTEM
Phil Vanek

Project commercial demand for cellular therapies will need to be met by a combination of new manufacturing approaches and supply chain management strategies. Today’s manufacturing models need to be further developed and modeled to meet that demand. This presentation will explore:

- Current manufacturing models
- Scaling challenges
- Modeling approaches
- Future factory design
- Logistics and supply chain

COMMERCIAL ASSESSMENT OF PRIVATE CELL AND GENE THERAPIES FROM INVESTOR PERSPECTIVE
Geoff MacKay

- Assessing risk
- Attractive segments
- Ensuring exits

SERUM-FREE MEDIA REQUIREMENTS DIFFER GREATLY BETWEEN MONOLAYER AND MICROCARRIER CULTURES
Steve Oh

- Current methods of mesenchymal stromal cell (MSC) production rely on traditional monolayer cultures in serum supplemented growth media. The projected demand of high...
cell numbers for cell therapy will require the development of scalable suspended microcarrier cultures in serum free media.

- We cultured seven different MSC lines, each in six different serum free media formulations in both monolayer and microcarrier cultures.
- Correlation between MSC growth in serum free media on monolayers vs microcarriers varied between R2 of 0.5 to 0.8. Thus, current serum-free media developed for monolayer cultures generally does not support proliferation in microcarrier cultures.
- Optimization of serum free media in microcarrier suspension culture, will have to address each of these issues: Cell line selection, media supplement screening on microcarriers, screening of coatings on microcarriers and verification of performance in suspension cultures such as spinner flasks.

OVERCOMING CHALLENGES OF VOLUME REDUCTION AND CELL WASHING PROCESS IN CELL THERAPY MANUFACTURING
Sunil Mehta

- Current bioprocessing technologies have limited applications in cell therapy manufacturing as they are optimized for the manufacture of recombinant proteins.
- Fluidized bed centrifugation maximizes viable cell density and recovery while removing particulates and impurities.
- Closed processing is essential as cells are the product. Scalable technology ensures minimal process changes from clinical trials to commercialization. Automation reduces human errors and single-use eliminates the need for CIP/SIP.

Special Session Summaries

ISCT EARLY STAGE PROFESSIONALS GROUP
#ISCT_ESP – CAREER OPTIONS FOR YOUNG PROFESSIONALS: WHY MY JOB ROCKS

THURSDAY, MAY 28TH, 2015 • TIME: 0800 – 0900
Florentine Ballroom III/IV

Chair: Deborah Griffin

The field of Cellular Therapy is rapidly growing - every day seems to bring a new therapy or a new clinical trial. A variety of skills is needed to successfully translate any cellular therapy from the benchtop to the bedside, and therefore a corresponding number of diverse jobs are necessary to ensure that patients receive the best and most effective care. However, as an Early Stage Professional (ESP), finding the right career choice is overwhelming and the plethora of options – though actually extensive – seems limited. We'll share why we chose our careers, shed some light on our paths from student to professional, and why our jobs do, indeed, rock. Representing the laboratory, the clinic, industry and academia, there is something for everyone. Goals of this session include:

1. Understand the variety of jobs available in cellular therapy
2. Identify skills that will help develop expertise
3. Identify concrete steps that can be taken to build your CV

ESP – THE ACADEMIC CELL THERAPY LABORATORY DIRECTOR
Patrick Hanley

- Learn why to be optimistic about options in cell therapy
- Identify the (many) advantages of training as a laboratory director

CAREER OPTIONS FOR ESPS: LEAVING THE LAB
Emily Culme-Seymour

- This talk will cover a few different career options open to ESPs who may want to take a step away from the lab bench
- Specifically, current roles, background and training, and challenges along the way will be discussed
- The aim of this session is to provide a forum for discussion for any ESPs facing the challenge of choosing their career path

WHY MY JOB ROCKS: TRANSLATIONAL RESEARCH IN ONCOLOGY
Saar Gill

- Describe the path from medical school to training in hematology-oncology
- Outline the decisions that led to post-specialization PhD in tumor immunology
- Explain how putting clinical training together with bench research lead to a career in translational immune-oncology, where I now serve as principal investigator on a number of pilot clinical trials that arose from my own laboratory’s research

PHYSICIAN ASSISTANTS: AT THE FOREFRONT OF PATIENT CARE
Tobi Fisher

- Learn about the Physician Assistant profession and education
- Physician Assistants improving access to healthcare
• Benefits of the Physician Assistant profession
• Physician Assistant role in the field of cellular therapy

ISCT PRESIDENTIAL TASK FORCE ON THE USE OF UNPROVEN CELLULAR THERAPIES ROUND TABLE: CONNECTING OUR STAKEHOLDERS, COMMUNICATING KNOWLEDGE, TRANSLATING THE PROVEN

FRIDAY, MAY 29TH, 2015 • 1230 - 1400
Roman Ballroom

Chair: Massimo Dominici (IT)
Co-Chairs: Aaron Levine, Karen Nichols, and John Rasko
Discussants from the ISCT Presidential Task Force on Unproven Cell Therapies

This session introduces the ISCT initiative on unproven cellular therapies (UCT) and launches a dynamic web-based living document to be actively shared with other cell therapy professionals, scientific organizations and patient associations. The session will dissect and constructively debate issues related to the unproven use of cells in the clinic. Join us for a lively discussion on the impact of unproven cellular therapies, from their complex and evolving nature and their impact in the clinic and towards commercialization. Attention is also provided on manufacturing and on the different regulatory landscapes that are constantly challenged by these approaches at the global level. ISCT promotes and develops effective strategies for communication on UCT in the bio-medical field and in the field worldwide to ultimately support patients that are considering unproven cellular interventions as possible therapeutic options.

CYTOTHERAPY: HOW TO REVIEW PAPERS FOR PEER-REVIEWED JOURNALS

SATURDAY, MAY 30TH, 2015 • TIME: 1115 – 1215
Pompeian Ballroom I/II

Chair and Speaker: John Barrett

Advances, discoveries, observations and opinions in science and medicine are not validated until they are published. The process of peer review therefore critically underpins the quality of science and contributes to the solid extension of existing knowledge into new areas. As such the peer reviewer has a responsibility to ensure that what is written follows the norms of scientific writing, adequately describes the work and is free from conclusions that cannot be justified by the facts as they are presented. It is probably true that no paper submitted for review is perfect. It is always possible therefore to suggest changes that will make the work more readable, satisfy obvious questions, and clarify important points. Furthermore, while the vast majority of papers submitted to journals are honest attempts at publication of data and work correctly conducted the possibility of the misdemeanors of plagiarism, misrepresentation and plain scientific fraud should never be disregarded. Thus the reviewer has a responsibility not only to help authors improve their manuscript (whether or not it is ultimately accepted for publication by the journal), as well as to ascertain as much as is possible the veracity of the work and the honesty of the individuals submitting the paper. Good reviewers succeed when they address the four issues: 1) How much does the work advance the field? 2) Does the data supplied support the conclusions? 3) How is the paper presented? What could be done to increase its quality? 4) Is this a paper that should be published – in its present form; with minor or major changes; or rejected? Here we discuss the ways to review a paper submitted for publication not only to provide the rigor that is needed but also to help the authors improve on their manuscript.

FEATURE PRESENTATION BY NASA:
STEM CELL-BASED TISSUE REGENERATIVE HEALTH IN MICROGRAVITY

SATURDAY, MAY 30TH, 2015 • TIME: 1250 – 1345
Roman Ballroom

Chair and Speaker: Eduardo Almeida

Mechanical unloading of stem cells during spaceflight in microgravity can alter their physiology and interfere with normal tissue regenerative processes. In the newt tail tissue regeneration model, we observed that the transition between the highly proliferative BrdU-positive blastema stem cell stage, to non-proliferating differentiated tissues was inhibited in microgravity resulting in smaller more immature tail regenerates with greater stemness. Using mouse bone marrow mesenchymal stem cell and mouse bone osteoprogenitor models in vivo, we observed that microgravity inhibited the appearance of mesenchymal stem cell differentiation gene expression markers, and induced p21/CDKN1a-mediated cell cycle arrest in the osteoblastic lineage. Finally, using differentiating embryoid bodies derived from mouse embryonic stem cells, we determined that tissue germ layer gene expression markers also fail to appear normally in microgravity, while stemness markers are retained. In total, these results suggest mechanical unloading in microgravity interferes with stem cell proliferation and differentiation patterns required for normal tissue regeneration, and highlight the potential importance of gravity mechanical loading in maintaining tissue regenerative health on Earth.
**Meeting Directory**

**CAESARS PALACE**

- **Registration**
  - Promenade Foyer

- **Plenary Session**
  - Roman Ballroom

- **Speaker Services**
  - Genoa

- **Concurrent Sessions**
  - Pompeian Ballroom, Florentine Ballroom, Capri and Roman Ballroom

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