ISCT 2018

Montréal · Canada · May 2-5

CONFERENCE PROGRAM

International Society
ISCT
Cell & Gene Therapy

www.isct2018.com

2019 ISCT melbourne
MAY 29–JUNE 1

save THE date

MAY 29–JUNE 1
2019

International Society
ISCT
Cell & Gene Therapy

celltherapysociety.org

International Society ISCT Cell & Gene Therapy

Table of Contents

Congrès annuel de l'International Society for Cellular Therapy

Mot pour le programme des participants 2018

Reconnu comme un important pôle de recherche scientifique internationale, le Québec dispose de chercheurs et d'organisations qui figurent parmi les plus réputés au monde. Il offre également un environnement exceptionnel pour le développement de la recherche dans le domaine de la santé.

Les activités de recherche et d'innovation s'accompagnent d'une haute responsabilité : celle de porter les espoirs d'un monde meilleur. Conséquemment, l'apport des chercheurs, de la relève scientifique et des innovateurs est précieux et considérable.

Nous en sommes tous conscients, le 21e siècle est annonciateur de changements majeurs dans le monde. C'est dans ce contexte que le gouvernement du Québec a mis en place la Stratégie québécoise des sciences de la vie 2017-2027, laquelle vise à accroître les investissements en recherche et en innovation; à favoriser la création d'entreprises innovatrices dans le secteur des sciences de la vie et à assurer leur croissance; à attirer de nouveaux investissements privés et à veiller à l'intégration des innovations dans le système de la santé et des services sociaux.

Beaucoup d'efforts ont été déployés pour soutenir la réalisation de projets en soins de santé personnalisés et stimuler la recherche clinique précoce. Par exemple, le Fonds de recherche du Québec – Santé a accordé une subvention au réseau de thérapie cellulaire et tissulaire ThéCell, qui vise à promouvoir la recherche translationnelle en thérapie cellulaire et tissulaire au Québec, avec comme objectifs une visibilité et un rayonnement internationaux.

Par ailleurs, plusieurs découvertes majeures dans le domaine de la médecine de précision ont mené et mèneront au développement de nouveaux traitements ou faciliteront la prise de décisions quant à la gestion de la maladie.

Le Québec a également mis en œuvre la Stratégie québécoise de la recherche et de l'innovation 2017-2022, qui s'articule autour de trois axes d'intervention visant à accroître la capacité de recherche et à soutenir l'innovation sous toutes ses formes : appuyer les chercheurs et les innovateurs pour assurer le foisonnement des idées; investir dans la recherche collaborative et les projets innovants; et assurer une recherche de qualité en maintenant l'accès à des infrastructures compétitives et leur financement.

Nous souhaitons que les activités auxquelles vous participerez à l'occasion de ce 24e congrès annuel de l'International Society for Cellular Therapy soient enrichissantes et renforcent les collaborations internationales, un aspect incontournable à l'avancement de la science et des sociétés.

La vice-première ministre,
ministre de l'Économie, de la Science et de l'Innovation
et ministre responsable de la Stratégie numérique,

Dominique Anglade

Le ministre de la Santé et des Services sociaux,

Gaétan Barrette

Annual Meeting of the International Society for Cellular Therapy

Message for 2018 Program

Recognized as a leading international hub of scientific research, Québec is home to some of the world's finest researchers and organizations. Québec is also an exceptional environment for health research development.

Research and innovation come with great responsibility: that of providing hope of a better world. To create this better world, experienced researchers, young scientists, and innovators all make invaluable and considerable contributions.

As we all know, the 21st century is ushering in major changes. This is why the Government of Québec launched the 2017-2027 Québec Life Sciences Strategy, which aims to increase investment in research and innovation, foster the creation of innovative companies in the life sciences sector and their growth, attract new private investment, and integrate innovation into the health and social services system.

Much has been done to support projects in personalized health care and to stimulate early clinical research. For example, the Fonds de recherche du Québec – Santé has given a grant to the ThéCell network, which promotes translational research in cell and tissue therapy in Québec to increase the visibility of our research and raise our international profile.

Major discoveries in precision medicine have led and will lead to new treatments or better decision-making for disease management.

Québec has also launched the 2017-2022 Québec Research and Innovation Strategy, which aims to increase research capacity and support innovation in all its forms through three key guidelines: support researchers and innovators so that they can grow ideas, invest in collaborative research and innovative projects, and commit to quality research by maintaining access to competitive infrastructure and funding.

We hope that the 24th Annual Meeting of the International Society for Cellular Therapy gives you the chance to experience and strengthen your international collaborations, which we must forge if we are to advance both science and society.

Deputy Premier,

Minister of Economy, Science and Innovation
and Minister responsible for the Digital Strategy,

Dominique Anglade

Minister of Health and Social Services,

Gaétan Barrette

Québec
Bureau du scientifique en chef

Dear delegates, guests and participants,

As Chief scientist of Québec it is a great pleasure for me to welcome you to the 2018 Annual Meeting of the International Society for Cell Therapy, back in Canada for the first time since 2005. This conference is undoubtedly recognized world-wide as a major scientific event. We are happy to see the force of attraction of Quebec and more specifically Montreal for the lifeblood of science, research and creativity put to the service of the well-being of patients around the world.

Québec has recently developed ambitious strategies in research and innovation, for life sciences and digital technologies. Our objectives are clear : be world leaders in key sectors and in particular, in life sciences and artificial intelligence. The Québec Research Funds play a very important role in achieving our goals by focusing on training the next generation of researchers. Leverage our gray matter is our best guarantee of success.

These four days will be filled with substantive discussions, high-level conferences, meetings with scientists and leading representatives of the next generation of researchers and business people, a rich ecosystem that revolves around the field of cell, tissue and gene therapy. This pooling of intellectual resources of the various actors will result in the advances of tomorrow to treat diseases so far incurable.

Tomorrow's progresses to cure diseases considered incurable to date, will result from pooling the intellectual resources of the different actors. The Quebec's Government is more than ever engaged in supporting the development of advanced therapies like regenerative medicine and cell, tissue and gene therapies. Our contribution to this event is a clear demonstration of that commitment and is definitely a unique opportunity to feed your thirst for networking and knowledge.

Montréal and all Québec are happy to welcome you and wish you a great Meeting !

Rémi Quirion, OC, PhD, CQ, FRSC

Chief Scientist of Québec

Fonds de recherche – Nature et technologies
Fonds de recherche – Santé
Fonds de recherche – Société et culture
500, rue Sherbrooke Ouest, bureau 800
Montréal (Québec) H3A 3C6
Téléphone : 514 873-2114
Télécopieur : 514 873-8764
www.bcg.gouv.qc.ca

Welcome from the President

Welcome to Montréal! On behalf of ISCT and members of the global cell therapy community, it gives me enormous pleasure to welcome you to Montréal, Canada. We are anticipating an outstanding conference, as we exchange ideas, learn about new breakthroughs, renew friendships, meet new colleagues and have some fun!

ISCT has a rich history of meetings spanning numerous regions across the globe and this meeting in Canada is no exception illustrating our commitment to being a global society and our strong commitment to our international membership.

Our Organizing Committee, has performed a stellar job with the planning what we envision will be one of the best meetings ever! To provide a balanced vision and worldwide expertise for the attendees the co-Chairs for the Scientific Program ***David DiGiusto*** (Regional VP for North America)**,** ***Sarah Nikiforow*** and ***Denis-Claude Roy*** have made amazing efforts to align the Strategies for Commercialization Track and the Quality and Operations Track with speakers from the old world and the new, and from both hemispheres.

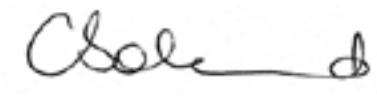
The diversity of our Society is a great strength, and accordingly, technologists, academics, manufacturing experts, clinicians, regulators, industry members, laboratory specialists, advanced practice practitioners, pharmacists, clinical research nurses and coordinators and all others integral to bring cell therapy from bench to bedside are here. Therefore, whatever your interest in the field of cell and gene therapy, I am confident that you will enjoy the conference and develop new insights into our ever expanding world of cellular and gene therapy.

With CAR-T cells in the spotlight, the Presidential Plenary will focus on Cancer Immunotherapy where we are fortunate to have attracted leaders in the field to speak including ***Stanley Riddell, Crystall Mackall*** and one of ISCT's Past Presidents, ***Robert Negrin***. While CAR-T cells were highlighted last year because of their FDA approval, we are mindful that ISCT members not in North America have their own regulatory systems and issues. We have hot topic sessions spanning regulatory T cell treatment of autoimmunity and applications in solid organ transplants, immunologic sculpting of HSCT and grafts, and oncolytic viruses, as well as the ever fertile area of mesenchymal stromal cell biology and therapy, and exciting new developments in the application of exosomes and gene modified immune cells. Sessions on natural killer cells will highlight progress in their clinical application with exciting new data.

A particular feature of ISCT 2018 is the number of pre-conference workshops with 6 concurrent tracks – the Global Regulatory Perspectives Workshop, the ISCT-FACT Cell Therapy Quality "Boot Camp", the ISCT-CBA Cord Blood Series (in partnership with ASBMT), the MSC Workshop on Potency and Clinical Efficiency, as well as the Cell Processing Track and a special Canadian Cell and Gene Therapy Strategy Workshop. All events are open to anyone registered for the Pre-Conference Day.

I think most of you will agree that every year the ISCT meetings move from strength to strength. Again we are excited to have 40+ oral abstract presentations at this meeting with our early stage professionals making a substantial contribution to these sessions. Both the oral and the poster sessions emphasize the very best in science, and we hope they will continue to generate stimulating discussions and new collaborations. In this year's program, you will find a variety of interesting sessions in our Quality and Operations and Strategies for Commercialization Tracks. Our industry participants are welcome partners, and this year there will be some highly informative corporate symposia and tutorials.

In closing, I would like to thank Dave, Denis-Claude, Sarah and the entire Organizing Committee as well as our incredible Head Office staff for all their extraordinary efforts leading up to our 24th Annual Meeting. In addition, we must also offer our enormous gratitude to our phenomenal industry partners and sponsors. Without their enduring support, this meeting would not be possible. We have an especially large number of exhibitors this year and I therefore look forward to meeting everyone in the exhibit hall and in the meeting rooms as we welcome each and every one of you to Montréal to ***Connect, Communicate, and Translate!***



Catherine M. Bollard, *MBChB, MD*
ISCT President, 2016-2018

Welcome from the Co-Chairs

"What you do with a discovery is create potential for further development."

– James Till, Gairdner International Award winner with Ernest McCulloch, (Canadians who discovered stem cells)

On behalf of the entire ISCT 2018 Organizing Committee, we would like to extend a warm welcome to Montréal to celebrate ISCT's 24th Annual Scientific Meeting. We are very excited to be holding this year's meeting in Montréal, Canada, one of North America's most vibrant and beautiful cities.

This year's meeting covers the latest and hottest areas of cell and gene therapy including cancer immunotherapy, mesenchymal stem cell biology and therapeutic development, exosome isolation and translation to clinical assessment, genome editing and iPSC biology, especially projects imminently heading to or already in the clinic. The conference opening highlight will be the Presidential Plenary which will focus on the breadth of cancer immunotherapies, including CAR T cells. Each of the 6 plenaries also generates its own specialist "plenary breakout" sessions, in order to be more broadly educational and to feature detailed presentations and oral abstracts relevant to the field.

Again this year, we have scheduled early morning "Hot Topics" sessions which will provide a more intimate, less formal setting to encourage interaction between presenters and the audience on topics such as immunologic HSCT graft sculpting and induction of tolerance along with "Scientific Areas of Focis" such as Stem cell expansion and Natural Killer cell applications. The stalwart tracks of Regulatory, Quality/Operations, and Strategies for Commercialization remain central to the conference. For 2018, we have featured Oral Abstract presentations through the ISCT Chief Scientific Officer's Abstract Showcase, Organizing Committee Abstract Showcase, and Young Investigator Abstract sessions in addition to the abstracts featured in the plenary breakout sessions. As always, the high quality of poster presentations will provide a stimulating opportunity to interact with fellow delegates.

In addition to the core conference program, there will be an extensive set of pre-conference workshops on Wednesday, May 2nd. These include workshops on Cell Processing with specific sections on novel cell processing technologies, contemporary approaches to quality control release testing and two sessions on cell product characterization with presentations from academic, industrial and FDA personnel. We are also proud to offer our highly successful pre-conference tracks on Global Regulatory Perspectives, MSC Potency and Clinical Efficacy, and a dedicated Cord Blood Workshop in partnership with the Cord Blood Association (CBA) and American Society for Blood and Marrow Transplantation (ASBMT), as well as a Cell Therapy Quality Bootcamp in partnership with FACT. New this year, the Canadian Cell & Gene Therapy Workshop, in partnership with CellCAN, will discuss academic and industry manufacturing infrastructure, regulation, funding, and reimbursement issues. This year, we have 42 oral abstract presentations and invited speakers from more than 20 countries, making this a truly international forum.

This scientific and clinical energy is going to further amplified by our location in the heart of Montréal, a vibrant, dynamic, multicultural and lively city known worldwide for its "joie de vivre"! Canada, and Montréal in particular, are perfectly aligned with the motto of the ISCT: Connect, Communicate, Translate. The cell therapy ecosystem is growing at an incredibly fast pace, and we are assembling the right people to cross-pollinate and continue that swift expansion. Montréal's open and warm atmosphere will inspire you, allow you to find new collaborators, and ensure that you are linking with the right business partners.

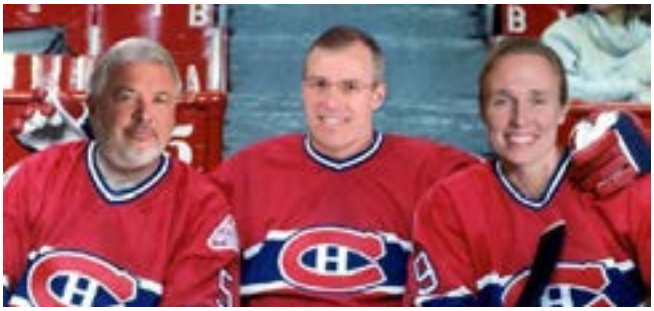
We welcome you to get acquainted with Montréal's culinary offerings (from decadent poutine to more sophisticated cuisine), museums, architecture, and nightlife. Montréal is also known as a fully bilingual city, mixing French, English and many other languages and cultures that enrich its environment. Finally, don't forget to enroll in the inaugural ISCT 5K run, which will be held in Old Montréal along the beautiful St. Lawrence River.

As always, we are exceedingly grateful for the support of our sponsors for the ISCT 2018 Annual Meeting. With unparalleled representation in the exhibit hall, which sold out months in

advance, we encourage attendees to visit the exhibitors, attend corporate symposia, tutorials and watch presentations in the Product Theatre, conveniently located in the exhibit hall.

We wish to thank all those who so enthusiastically took part in the organization of the conference. We have been delighted by the commitment of fellow ISCT members, who, in spite of early morning and late-night conference calls, have shown exceptional eagerness in planning with us such an ambitious, power-packed meeting. Our thanks also go out to all the speakers who have generously agreed to share their experiences during the conference. Thanks are also in order for ISCT Head Office, tirelessly working to generate an outstanding meeting. We look forward to the culmination of the above efforts and enthusiasm!

Bienvenue à Montréal!



David DiGiusto, *PhD*
Stanford Healthcare/Stanford School of Medicine, United States

Denis-Claude Roy, *MD, FRCPC*
CellCAN, Université de Montréal, Hôpital Maisonneuve-Rosemont, Canada

Sarah Nikiforow, *MD, PhD*
Dana Farber Cancer Institute United States

Organizing Committee

Meeting Co-Chairs

David DiGiusto, ***PhD***
Stanford Healthcare and Stanford School of Medicine
United States

Sarah Nikiforow, ***MD, PhD***
Dana-Farber Cancer Institute
United States

Denis-Claude Roy, ***MD, FRCPC***
CellCAN, Maisonneuve-Rosemont Hospital, University of Montréal, Canada

Organizing Committee Members

Rosemarie Bell, ***BAppSc Micro/Biochem MASM***
Co-Chair, Quality & Operations Track
QIMR Berghofer Medical Research Institute, Australia

Rachele Ciccocioppo, ***MD***
University of Verona Department of Medicine, Italy

Jacques Galipeau, ***MD***
University of Wisconsin-Madison
United States

David Gottlieb, ***MBBS, MD, FRACP FRCPA***
Westmead Hospital, University of Sydney, Australia

Armand Keating, ***MD***
University of Toronto
Canada

Donald Kohn, ***MD***
University of California, Los Angeles
United States

Sai Kiang Lim, ***PhD***
*Institute of Medical Biology (IMB) Agency for Science and Technology (A*STAR), Singapore*

William Milligan, ***BSc***
Chair, Strategies for Commercialization Track
Steminent Biotherapeutics Inc
Canada/Taiwan/United States

Sandeep Soni, ***MD***
Stanford University
United States

Duncan Stewart, ***MD***
The Ottawa Hospital Research Institute, Canada

Sowmya Viswanathan, ***PhD***
Co-Chair, Quality & Operations Track
University Health Network, University of Toronto, Canada

Ex-Officio

Catherine Bollard, ***MBChB, MD***
Children's National Medical Center and The George Washington University
United States

John Rasko, ***AO, BSc(Med), MBBS(Hons), PhD, MAICD, FFSc(RCPA), FRCPA, FRACP, FAHMS***
Royal Prince Alfred Hospital, Australia

Daniel J. Weiss, ***MD, PhD***
University of Vermont School of Medicine
United States

Strategies for Commercialization Track Subcommittee

Chair:

William Milligan, ***BSc***
Steminent Biotherapeutics Inc, Canada/Taiwan/United States

Members:

Julie Allickson, ***PhD***
Wake Forest Institute for Regenerative Medicine, United States

Gerhard Bauer, ***PhD***
University of California Davis, United States

Dawn Driscoll, ***MBA, PhD***
Cell Therapies PTY, Australia

Simon Ellison, ***MBA***
World Courier, United Kingdom

Miguel Forte, ***MD, PhD***
Zelluna Immunotherapy, Belgium

Uri Herzberg, ***PhD, MBA***
Celularity, United States

Ohad Karnieli, ***MBA, PhD***
ATVIO Biotechnology, Israel

Bruce Levine, ***PhD***
University of Pennsylvania, United States

Robert (Willie) Mays, ***PhD***
Athersys, United States

Michael Mendicino, ***PhD***
Hybrid Concepts International, United States

Julie Murrell, ***PhD***
MilliporeSigma, United States

Colin Lee Novick
CJ Partners, Japan

Quality and Operations Track Subcommittee

Co-Chairs:

Rosemarie Bell, ***BAppSc Micro/Biochem MASM***
QIMR Berghofer Medical Research Institute, Australia

Sowmya Viswanathan, ***PhD***
University Health Network, University of Toronto, Canada

Members:

Shirley Bartido, ***PhD, MBA***
Cellectis Inc., United States

Christopher Bravery, ***PhD***
Consulting on Advanced Biologicals Ltd., United Kingdom

Heather Garrity, ***MHA***
Dana Farber Cancer Institute, United States

Aisha Khan, ***MSc, MBA***
University of Miami Interdisciplinary Stem Cell Institute, United States

Nadim Mahmud, ***MD, PhD***
University of Illinois Hospital and Health Sciences System, United States

Gabrielle O'Sullivan, ***PhD, MPH***
Royal Prince Alfred Hospital, Australia

GENERAL CONFERENCE INFORMATION

Registration

TUESDAY, MAY 1	16:00–19:00	Level 5 Foyer
WEDNESDAY, MAY 2	07:00–20:30	Level 5 Foyer
THURSDAY, MAY 3	07:00–19:00	Level 5 Foyer
FRIDAY, MAY 4	07:00–18:00	Level 5 Foyer
SATURDAY, MAY 5	07:30–15:00	Level 5 Foyer

INCLUDED IN YOUR ANNUAL MEETING REGISTRATION FEE:

- Access to the President's Welcome Address and Exhibit Open House on May 2
- Access to all scientific and educational sessions excluding pre-conference events
- Access to the exhibit hall during all opening hours
- Lunch, all coffee breaks and refreshments served from May 3-5
- Conference Program Book
- 2018 Abstract Supplement of Cytotherapy, the official journal of ISCT
- Access to the Conference App
- Delegate Bag
- Access to presentations online (post-event)

EXHIBIT-ONLY ATTENDEES RECEIVE:

- Access to the Exhibit Hall during all opening hours including the President's Welcome Address and Exhibit Open House on May 2
- Access to all Corporate Sessions
- Conference meals and refreshments served in the exhibit hall May 2-4
- Conference Program Book
- Access to the Conference App

Exhibit Hall Hours

WEDNESDAY, MAY 2	19:30–21:30
THURSDAY, MAY 3	09:00–19:30
FRIDAY, MAY 4	09:00–18:30

WiFi

Available throughout the ISCT Annual Meeting Space

NETWORK NAME: ISCT2018
PASSWORD: KEYBIOMTL

Conference and Partnering App

SCHEDULE PARTNERING MEETINGS AND CONNECT WITH OTHER DELEGATES

- View the program, abstracts, speaker bios, sponsor/exhibit directory, and much more
- Build your itinerary
- Search "ISCT" in your app store to download the conference app.

Twitter

Join the conversation on Twitter!
#ISCT2018

Social Events

WEDNESDAY, MAY 2
ISCT 2018 President's Welcome and Exhibit Open House

ADMISSION:

Free for all conference delegates

WHERE & WHEN:

Wednesday, May 2

19:00–19:30 President's Welcome Address in the Plenary Hall

19:30–21:30 ISCT 2018 Welcome Reception and Exhibit Open House in the Exhibit Hall

Wine and hors d'oeuvres will be served
Sponsored in part by Northern Therapeutics

THURSDAY, MAY 3
Poster Session Reception #1

Open to all conference delegates

WHERE & WHEN:

Thursday, May 3
18:00–19:30 in the Poster Hall

ISCT 2018 Early Stage Professionals (ESP) Networking Reception

By Invitation Only

WHERE & WHEN:

Thursday, May 3
19:00–21:00 in the Westin Montréal Presidential Suite

Sponsored by MaxCyte

ISCT 2018 Industry Networking Event

By Invitation Only

WHERE & WHEN:

Thursday, May 3
19:30–21:30 Offsite

Supported by CellCAN and the Canadian Pavilion Partners

FRIDAY, MAY 4
ISCT 2018 5K Run the River Event

ENTRY:

$25 Registration Fee – Purchase at Registration Desk

WHERE & WHEN:

Friday, May 4

06:15 Meet in front of Embassy Suites Hotel

06:30 Run Begins

Poster Session Reception #2

Open to all conference delegates

WHERE & WHEN:

Friday, May 4
17:00–18:30 in the Poster Hall

ISCT 2018 Gala Event

ADMISSION:

$135USD ISCT Members

$95USD ISCT Member Technologists, APPs*, Trainees, Students, and Emerging Economies

$165USD Non-Members

Purchase at Registration Desk while Supplies Last

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

WHERE & WHEN:

Friday, May 4
19:00–23:00: Le Windsor Ballrooms
1170 Rue Peel #110, Montréal, QC, H3B 4P2
Bus transportation available from the Westin Hotel starting at 18:30, please see Registration Desk for details

Continuing Medical Education Credits

Satisfactory Completion

Learners must complete an evaluation form to receive a certificate of completion. You must participate in the entire activity as partial credit is not available. If you are seeking continuing education credit for a specialty not listed below, it is your responsibility to contact your licensing/certification board to determine course eligibility for your licensing/certification requirement.

PHYSICIANS

In support of improving patient care, this activity has been planned and implemented by Amedco LLC and International Society for Cellular Therapy. Amedco LLC is jointly accredited by the Accreditation Council for Continuing Medical Education (ACCME), the Accreditation Council for Pharmacy Education (ACPE), and the American Nurses Credentialing Center (ANCC), to provide continuing education for the healthcare team.

Credit Designation Statement – Amedco LLC designates this live activity for a maximum of **19.25 *AMA PRA Category 1 Credits*™.** Physicians should claim only the credit commensurate with the extent of their participation in the activity.

How to get your CE Certificate:

Go to ISCT.CmeCertificateOnline.com

1. Click on the "ISCT 2018 Annual Meeting" link
2. Questions? Email Certificate@AmedcoEmail.com

Continuing Medical Laboratory Education (CMLE) Credits

This continuing medical laboratory education activity is recognized by the American Society for Clinical Pathology as meeting the criteria for 19.25 hours of CMLE credit. ASCP CMLE credit hours are acceptable to meet the continuing education requirements for the ASCP Board of Registry Certification Maintenance Program.

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, Plenary Breakout Sessions, Hot Topic Sessions, Advanced Practice Professionals Sessions, Scientific Areas of Focus Sessions, and Quality and Operations Track Sessions from May 3-5, 2018.

In order to receive credit for this activity, participants must complete online evaluations for the sessions they attend. Please visit www.isct2018.com to complete the evaluation form. Online evaluation must be completed **by June 17, 2018.**

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

Disclosure of Conflict of Interest

The following table of disclosure information is provided to learners and contains the relevant financial relationships that each individual in a position to control the content of CME disclosed to Amedco. All of these relationships were treated as a conflict of interest, and have been resolved. (C7 SCS 6.1 – 6.2, 6.5)

FIRST	LAST	COMMERCIAL INTEREST
Juliet	Barker	NOHLA: Consultant Angiocrine Biotec: Research Grant Overall Principal Investigator Gamida: Consultant
John	Bell	Turnstone Biologics: Founder and Equity Share Holder
Gilbert	Bernier	StemAxon: Corporate Board Member
Catherine	Bollard	Cellectis: Scientific/Medical Advisory Board Member Neximmune: Scientific/Medical Advisory Board Member Torque: Scientific/Medical Advisory Board Member

FIRST	LAST	COMMERCIAL INTEREST
Alan	Burns	Takeda Pharmaceuticals: Employee
Chulhee	Choi	Cellex Life Sciences, Incorporated:Founder
Randolph	Corteling	ReNeuron: Employee
Colleen	Delaney	Nohla Therapeutics: Employee Nohla Therapeutics: Stock Shareholder Nohla Therapeutics: Scientific/Medical Advisory Board Member Biolife Solutions: Scientific/Medical Advisory Board Member
Jacques	Galipeau	Cambium Medical Technologies, LLC: Stock Shareholder
Bambi	Grilley	Lokon Pharma AB: Consultant
Marie-Josée	Hébert	Fisher Technologies: Patent Holder
Helen	Heslop	Viracyte: Founder Marker Therapeutics: Founder Novartis: Scientific/Medical Advisory Board Member Tessa Therapeutics: Research Grant Site Principal Investigator Cell Medica: Research Grant Site Principal Investigator
Michael	Holmes	Sangamo Therapeutics, Inc.: Employee
Timothy	Kieffer	enGene Inc: Co-Founder and Patent Holder
Donald	Kohn	Orchard Therapeutics: Scientific/Medical Advisory Board Member
Sai Kiang	Lim	Paracrine Therapeutics: Founder
Rainer	Marksteiner	Innovacell Biotechnologie AG: Employee
Everett	Meyer	GigaGen: Stock Shareholder Triursus Therapeutics: Stock Shareholder
Maria	Mirotsou	Astellas: Employee
Robert	Negrin	Amgen: Consultant
Sarah	Nikiforow	Kite Pharma: Scientific/Medical Advisory Board Member
Douglas	Olson	Buhlmann Diagnostics Corp: Corporate Board Member
Bruno	Peault	Lipogems: Other Financial or Material Support
Matthew	Porteus	CRISPR Therapeutics: Founder CRISPR Therapeutics: Consultant
Mahendra	Rao	Mahendra Rao LLC: Consultant
John	Rasko	BlueBird Bio: Research Grant Site Principal Investigator Spark Therapeutics: Research Grant Site Principal Investigator Genea: Stock Shareholder Imago Therapeutics: Consultant Rarecyte: Stock Shareholder FSHD Global Research: Corporate Board Member
Stanley	Riddell	Juno Therapeutics: Founder, Adaptive Biotechnology: Scientific/Medical Advisory Board Member Nohla: Scientific/Medical Advisory Board Member
Denis-Claude	Roy	Kiadis Pharma: Research Grant Overall Principal Investigator Kiadis Pharm: Patent Holder SpecificiT: Research Grant Overall Principal Investigator Novartis: Consultant Jazz: Consultant
Andrew	Scharenberg	Casebia Therapeutics: Employee
Khalid	Shah	AMASA Technologies: Founder
Sandeep	Soni	Crispr Therapeutics: Consultant
Duncan	Stewart	Northern Therapeutics: Advisor Lung Rx: Other Financial Support
Jean	Tang	Pellepharm: Founder
John	Zaia	bluebird bio: Consultant

ISCT Leadership

ISCT Global Executive Committee

Catherine Bollard, ***MBChB, MD***
President, June 2016 – June 2018

John Rasko, ***AO, BSc(Med), MBBS(Hons), PhD, MAICD, FFSc(RCPA), FRCPA,FRACP, FAHMS***
President–Elect, June 2016 – June 2018

Massimo Dominici, ***MD***
Past President, June 2016 – June 2018

Lizette Caballero, ***BSc, MLS(ASCP)CM***
Global Secretary, June 2016 – June 2019

Emily Culme–Seymour, ***PhD***
Global Treasurer, June 2017 – June 2020

Janet Macpherson, ***PhD***
Australia & New Zealand, Regional Vice–President, June 2016 – June 2018

Mark Lowdell, ***PhD, FRCPath, FRSB***
Europe, Regional Vice–President, June 2016 – June 2018

Satoshi Takahashi, ***MD, PhD***
Asia, Regional Vice–President, June 2017– June 2019

David DiGiusto, ***PhD***
North America, Regional Vice–President, June 2016 – June 2018

Maroun Khoury, ***PhD***
South & Central America, Regional Vice–President, June 2017 – June 2019

Daniel J. Weiss, ***MD, PhD***
Chief Scientific Officer, June 2016 – June 2019

Miguel Forte, ***MD, PhD***
Chief Commercialization Officer, June 2016 – June 2018

Karen Nichols, ***Esq.***
Chief Regulatory Officer, March 2016 – March 2019

Rosemarie Bell, ***BAppSc Micro/Biochem MASM***
Co–Chair, Lab Practices Committee, June 2017 – June 2018

John Barrett, ***MD***
Senior Editor of the Journal, July 2014 – July 2018

Queenie Jang, ***BSc (Pharmacy), MBA***
CEO

ISCT Regional Executive Committees

Asia Regional Executive Committee

Satoshi Takahashi, ***MD, PhD***
Asia, Regional Vice-President, June 2017 – June 2019

Oscar Lee, ***MD, PhD***
Asia, Regional Vice-President Elect, June 2017 – June 2019

Melissa Lin, ***PhD***
Asia, Regional Treasurer, June 2017 – June 2020

Pawan Kumar Gupta, ***MBBS, MD, DNB, PhD***
Asia, Regional Secretary, June 2017 – June 2020

Australia and New Zealand Regional Executive Committee

Janet Macpherson, ***PhD***
Australia & New Zealand, Regional Vice-President, June 2016 – June 2018

Ngaire Elwood, ***PhD***
Australia & New Zealand, Regional Vice-President Elect, June 2016 – June 2018

Vicki Antonenas, ***BSc, MSc***
Australia & New Zealand, Regional Secretary, June 2017 – June 2020

Cheryl Hutchins, ***BSc (Hons I), PhD***
Australia & New Zealand, Regional Treasurer, June 2017 – June 2020

Europe Regional Executive Committee

Mark Lowdell, ***PhD, FRCPath, FRSB***
Europe, Regional Vice-President, June 2016 – June 2018

Jaap Jan Boelens, ***MD, PhD***
Europe, Regional Vice-President Elect, June 2016 – June 2018

Mohamed Abou-El-Enein, ***MD***
Europe, Regional Secretary, June 2017 – June 2020

Britta Eiz-Vesper, ***PhD***
Europe, Regional Treasurer, June 2017 – June 2020

North America Regional Executive Committee

David DiGiusto, ***PhD***
North America, Regional Vice-President, June 2016 – June 2018

Lynn O'Donnell, ***PhD***
North America, Regional Vice-President Elect, June 2016 – June 2018

Sarah Nikiforow, ***MD, PhD***
North America, Regional Secretary, June 2017 – June 2020

Patrick Hanley, ***PhD***
North America, Regional Treasurer, June 2017 – June 2020

South and Central America Regional Executive Committee

Maroun Khoury, ***PhD***
South & Central America, Regional Vice-President, June 2017 – June 2019

Patricia Rocco, ***MD, PhD***
South & Central America, Regional Vice-President Elect, June 2017 – June 2019

Jhann Arturo, ***MD, MSc, PhD***
South & Central America, Regional Secretary, June 2017 – June 2020

Hector Mayani, ***PhD***
South & Central America Regional Treasurer October 2017 - June 2020

ISCT Advisory Board

Massimo Dominici, ***MD***
Chair (Past President), June 2016 – June 2018

Edwin Wagena, ***MSc, PhD***
Member (Europe, Past Regional Vice–President), June 2016 – June 2018

Steve Oh, ***PhD***
Member (Asia, Past Regional Co Vice–President), June 2017 – June 2019

Anish Majumdar, ***PhD***
Member (Asia, Past Regional Co Vice–President), June 2017 – June 2019

Gustavo Moviglia, ***MD, PhD***
Member (South & Central America, Past Regional Vice–President), June 2017 – June 2019

William Janssen, ***PhD***
Member (North America, Past Regional Vice–President), June 2016 – June 2018

Robert Nordon, ***PhD***
Member (Australia & New Zealand, Past Regional Vice–President), June 2016 – June 2018

Edwin Horwitz, ***MD, PhD***
Elected Member MD, June 2017 – June 2019

Ping Law, ***PhD***
Elected Member PhD, June 2016 – June 2018

Rosemarie Bell, ***BAppSc Micro/Biochem MASM***
Elected Member Technologist, June 2016 – June 2018

Athena Russell, ***BSc, MT (AAB)***
Elected Member Technologist, June 2017 – June 2019

All members of the ISCT Global Executive Committee are also ex officio members of the ISCT Advisory Board.

ISCT 2018 Program at a Glance

Wednesday, May 2

07:00–20:30	Registration					*Level 5 Foyer*
07:00–18:00	Speaker Services					*Room 521C*
08:30–10:00	**MSC WORKSHOP SESSION 1** Development of Potency Assays to Predict MSC Clinical Efficacy *Room 520CF*	**CELL PROCESSING TRACK SESSION 1** Novel Cell Processing Technologies *Room 520 BE*	**GRP WORKSHOP SESSION 1** Locally Developed Complex Immunotherapies for Global Supply *Room 520 AD* *8:00 start	**ISCT-FACT CT QUALITY BOOTCAMP SESSION 1** Quality Management Program *Room 519AB*	**ISCT-CBA CORD BLOOD SERIES IN PARTNERSHIP WITH ASBMT SESSION 1** Current Status of Cord Blood Transplantation *Room 518A*	**CANADIAN CGT STRATEGY WORKSHOP SESSION 1** Cell & Gene Therapy Manufacturing Infrastructure *Room 518BC*
10:00–10:15	Coffee Break					*Level 5 Foyer*
10:15–11:45	**MSC WORKSHOP SESSION 2 –** Methods to Improve MSC Engraftment and Potency In Vivo *Room 520CF* *Industry Luncheon*	**CELL PROCESSING TRACK SESSION 2** Growth Areas in Novel Cell Therapy Quality Control And Release Testing *Room 520 BE*	**GRP WORKSHOP SESSION 2** CMC Considerations When Expanding the Market into Other Regions *Room 520 AD* *Lunch Seminar presented by HESI* *Room 520 AD*	**ISCT-FACT CT QUALITY BOOTCAMP SESSION 2** Validation, Verification, And Qualification *Room 519AB*	**ISCT-CBA CORD BLOOD SERIES IN PARTNERSHIP WITH ASBMT SESSION 2** Cord Blood Immunotherapy *Room 518A*	**CANADIAN CGT STRATEGY WORKSHOP SESSION 2** Investment in Canada: The Global Advantage *Room 518BC*
11:45–13:00	Lunch					*Level 5 Foyer*
13:00–14:30	**MSC WORKSHOP SESSION 3 –** "Responders" To MSC Therapy - Predicting Clinical Outcome *Room 520CF*	**CELL PROCESSING TRACK SESSION 3** Cell Product Characterization Part I *Room 520 BE*	**GRP WORKSHOP SESSION 3** Trial Design and Patient Cohorts *Room 520 AD* *12:45 start	**ISCT-FACT CT QUALITY BOOTCAMP SESSION 3** Auditing *Room 519AB*	**ISCT-CBA CORD BLOOD SERIES IN PARTNERSHIP WITH ASBMT SESSION 3** Manufacturing Novel Cell Therapy Products from Cord and Blood Tissue *Room 518A*	**CANADIAN CGT STRATEGY WORKSHOP SESSION 3** Making Frontline Life-Saving Therapies Accessible & Economically Feasible *Room 518BC*
14:30–14:45	Coffee Break					*Level 5 Foyer*
14:45–16:15	**MSC WORKSHOP SESSION 4** Wrap Up Summary and Panel Discussion *Room 520CF*	**CELL PROCESSING TRACK SESSION 4** Cell Product Characterization Part II *Room 520 BE*	**GRP WORKSHOP SESSION 4** Biological Variation and Cell Product Efficacy *Room 520 AD* *16:45 end	**ISCT-FACT CT QUALITY BOOTCAMP SESSION 4** Occurrence Management *Room 519AB*	**ISCT-CBA CORD BLOOD SERIES IN PARTNERSHIP WITH ASBMT SESSION 4** Cord Blood for Regenerative Medicine *Room 518A*	**CANADIAN CGT STRATEGY WORKSHOP SESSION 4** Strengthening the Canadian RMCT Ecosystem *Room 518BC*
16:30–18:30	**CORPORATE SYMPOSIUM HOSTED BY MILTENYI BIOTEC**					*Room 520CF*
19:00–19:30	**PRESIDENT'S WELCOME ADDRESS**					*Plenary Hall*
19:30–21:30	**ISCT 2018 WELCOME RECEPTION AND EXHIBIT OPEN HOUSE**					*Exhibit Hall*

Thursday, May 3, 2018

07:00–19:00	Registration					*Level 5 Foyer*
07:00–18:00	Speaker Services					*Room 521C*
07:30–08:30	**HOT TOPIC SESSION 1** Tregs and Other Cell Subtypes in Autoimmune Disease *Room 520BE*	**HOT TOPIC SESSION 2** Approaches to Overcoming Limitations of Cell and Gene Therapies *Room 520CF*	**CORPORATE BREAKFAST TUTORIAL** GE Healthcare *Room 518BC*	**CORPORATE BREAKFAST TUTORIAL** Roche Diagnostics *Room 519AB*		
08:45–09:00	**OPENING REMARKS AND 2018 CAREER ACHIEVEMENT AWARD PRESENTATION**					*Plenary Hall*
09:00–10:30	**PLENARY SESSION 1** Presidential Plenary on Cancer Immunotherapy					*Plenary Hall*
10:30–11:00	Coffee Break with Exhibits **CORPORATE PRODUCT THEATRES HOSTED BY GE HEALTHCARE AND MILLIPORESIGMA**					*Exhibit Hall*
11:00–12:15	**PLENARY BREAKOUT 1** Cancer Immunotherapy I *Room 519AB*	**PLENARY BREAKOUT 2** Cancer Immunotherapy II *Room 520CF*	**APP TRACK SESSION 1** Pharmacy Roles in CT & HSC Transplant *Room 518A*	**Q&O* TRACK SESSION 1** Progress in the World of Standards for CGT *Room 520BE*	**SFC** TRACK SESSION 1** Future Proofing Your Supply Chain *Room 518BC*	**SFC TRACK SESSION 2** How do Clinical Trial Design and Learnings Impact Commercialization? *Room 520AD*
12:15–13:45	Lunch with Exhibits **CORPORATE PRODUCT THEATRES HOSTED BY BECKMAN COULTER, CCRM, MILTENYI BIOTEC, BE THE MATCH**					*Exhibit Hall*
12:30–13:30	**CORPORATE TUTORIAL HOSTED BY BD BIOSCIENCES**					*Room 518BC*
13:45–15:15	**PLENARY SESSION 2** Mesenchyme Biology and Translational Use					*Plenary Hall*
15:15–15:45	Coffee Break with Exhibits **CORPORATE PRODUCT THEATRE HOSTED BY IRVINE SCIENTIFIC**					*Exhibit Hall*
15:45–17:00	**PLENARY BREAKOUT 1** Endothelial Progenitor Cells *Room 519AB*	**PLENARY BREAKOUT 2** Translational Use of MSCs *Room 520CF*	**ISCT CSO ABSTRACT SHOWCASE** *Room 518BC*	**APP TRACK SESSION 2** CAR T Immunotherapy: Science and Logistics *Room 518A*	**Q&O TRACK SESSION 2** Applying Change Within Controlled Systems and Processes *Room 520BE*	**SCIENTIFIC AREA OF FOCUS** Stem Cell Biology and Expansion *Room 520AD*
17:00–18:30	**APP TRACK SESSION 3** What's New in Cord Blood Transplantation for Hematologic Malignancies					*Room 518A*
17:15–19:15	**CORPORATE SYMPOSIUM HOSTED BY NOVARTIS ONCOLOGY**					*Plenary Hall*
18:00–19:30	Poster Session 1					*Poster Hall*
19:00–21:00	**ISCT EARLY STAGE PROFESSIONALS NETWORKING SESSION** *(By Invitation Only)*					*Westin Presidential Suite*
19:30–21:30	**ISCT INDUSTRY NETWORKING RECEPTION** *(By Invitation Only)*					*Offsite*

**Quality and Operations Track ** Strategies for Commercialization Track*

Friday, May 4, 2018

06:30–07:30	**ISCT 2018 5K RUN THE RIVER EVENT**					*Offsite*
07:00–18:00	Registration					*Level 5 Foyer*
07:00–17:00	Speaker Services					*Room 521C*
07:30–08:30	**HOT TOPIC SESSION 3** Immunologic Sculpting of T Cell and Stem Cell Grafts *Room 520BE*	**HOT TOPIC SESSION 4** Oncolytic Viruses as Cancer Therapeutics *Room 520CF*	**CORPORATE BREAKFAST TUTORIAL** American Society of Mechanical Engineers (ASME) *Room 518BC*			
08:45–10:15	**PLENARY SESSION 3** Exosomes					*Plenary Hall*
10:15–10:45	Coffee Break with Exhibits **CORPORATE PRODUCT THEATRE HOSTED BY BIOLAMINA**					*Exhibit Hall*
10:45–12:15	**PLENARY BREAKOUT 1** Exosome Technology *Room 518A*	**PLENARY BREAKOUT 2** Lessons Learned During CAR T Commercial Roll Out at Clinical Sites *Room 520CF*	**ISCT PRESIDENTIAL TASK FORCE SESSION** *Room 519AB*	**Q&O* TRACK SESSION 3** Regulation of MTMMs and ATMPs *Room 520BE*	**SFC** TRACK SESSION 3** Tools and Automation Solutions for CGT Development *Room 518BC*	**SCIENTIFIC AREA OF FOCUS** NK Cell Therapy: A New Frontier Revisited *Room 520AD*
12:15–13:45	Lunch with Exhibits **CORPORATE PRODUCT THEATRES HOSTED BY COOK REGENTEC AND CELLCAN**					*Exhibit Hall*
12:30–13:30	**CORPORATE TUTORIAL HOSTED BY MACOPHARMA**					*Room 518BC*
13:45–15:15	**PLENARY SESSION 4** Reimagining Cancer Care and Delivering on the Promise of CAR-T Therapies					*Plenary Hall*
15:15–15:30	Coffee Break with Exhibits **CORPORATE PRODUCT THEATRE HOSTED BY AVENTACELL BIOMEDICAL**					*Exhibit Hall*
15:30–17:00	**PLENARY BREAKOUT 1** Pre-Clinical Application of Exosomes *Room 519AB*	**PLENARY BREAKOUT 2** Market Access & Reimbursement of CAR T Therapies *Room 520CF*	**ISCT 2018 ORGANIZING COMMITTEE ABSTRACT SHOWCASE** *Room 520AD*	**ESP SESSION 1** Translating CGT Products from Bench to Bedside *Room 518A*	**Q&O TRACK SESSION 4** Day to Day Operations of a Stem Cell Lab *Room 520BE*	**SFC TRACK SESSION 4** 21st Century Global Regulatory Impact on Commercialization *Room 518BC*
17:00–18:30	Poster Session 2					*Poster Hall*
17:15–17:45	**CORPORATE MASTER CLASS HOSTED BY THERMO FISHER SCIENTIFIC**					*Exhibit Hall*
19:00–23:00	**ISCT 2018 GALA** ***Tickets Required***					*Offsite* Buses leave from Westin Hotel at 18:30

*Quality and Operations Track
** Strategies for Commercialization Track

International Society ISCT Cell & Gene Therapy

Saturday, May 5, 2018

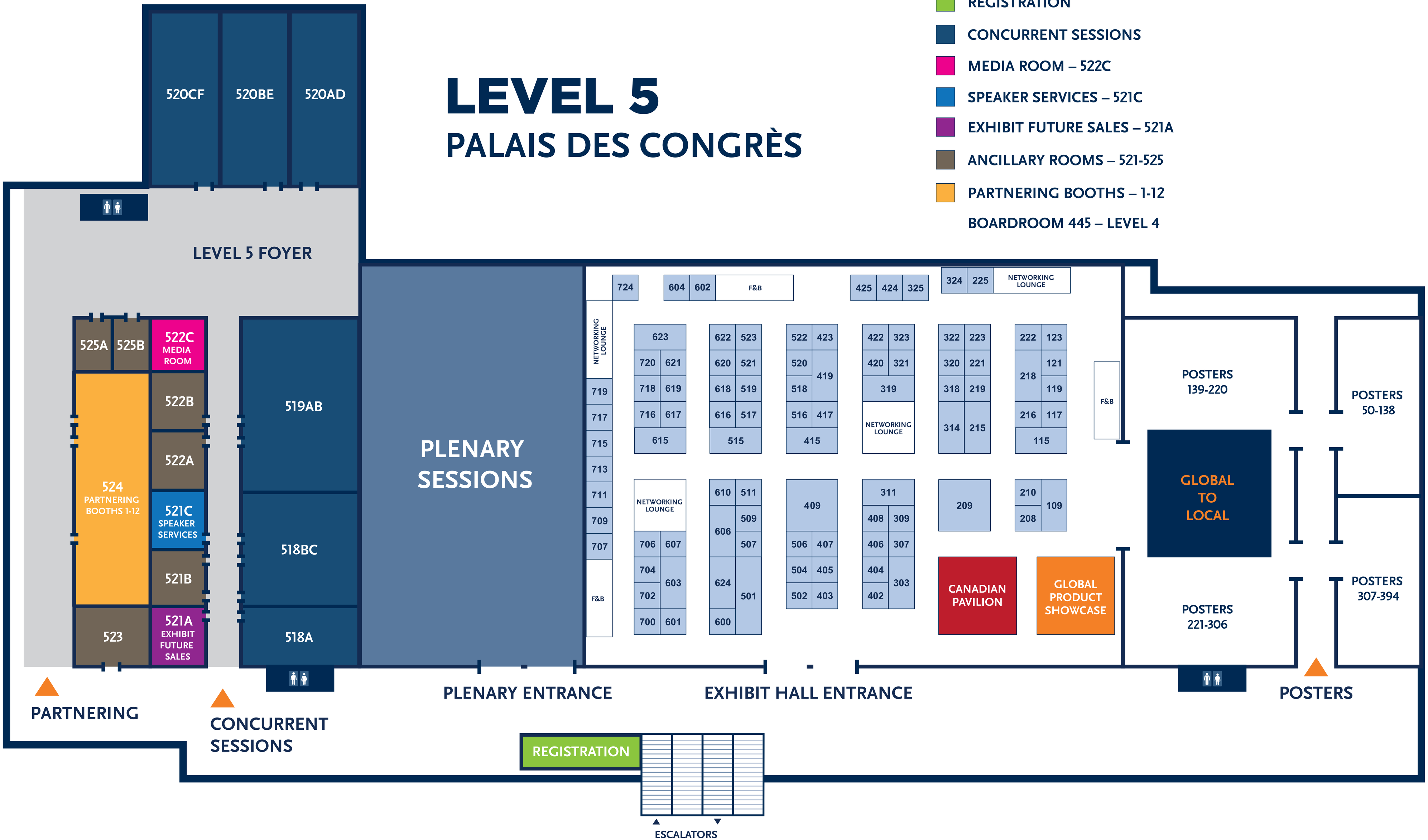
07:30–15:00	Registration and Speaker Services				*Level 5 Foyer and Room 521C*
08:00–09:00	**HOT TOPIC SESSION 5** Solid Organ Transplantation: Tolerance Induction and Tregs Immunotherapy *Room 519AB*	**HOT TOPIC SESSION 6** Biomaterials and Clinical Uses *Room 520CF*	**Q&O* TRACK SESSION 5** Data Integrity *Room 520BE*	**SFC** TRACK SESSION 5** Managing Patient Expectations *Room 518BC*	
09:15–10:45	**PLENARY SESSION 5** ISCT-ASGCT Joint Session: Genome Editing for Benign and Malignant Diseases				*Plenary Hall*
10:45–11:00	Coffee Break				*Level 5 Foyer*
11:00–12:00	**PLENARY BREAKOUT 1** Editing to Enhance Cell Therapy - Universal T Cells *Room 519AB*	**PLENARY BREAKOUT 2** Ex Vivo Editing - HSC and iPSC *Room 520CF*	**YOUNG INVESTIGATOR ABSTRACT SHOWCASE** *Room 520AD*	**Q&O* TRACK SESSION 6** Challenges of cGMP Clean Room Facility for Cell Therapy Manufacturing in an Academic Setting *Room 520BE*	**SFC** TRACK SESSION 6** Growing the Pie: Has Investor Interest Increased for CGT post-approval of Kymriah, Yescarta and Luxturna? *Room 518BC*
12:00–13:30	Lunch				*Level 5 Foyer*
12:15–13:15	**ISCT ANNUAL GENERAL BUSINESS MEETING**				*Plenary Hall*
13:30–15:00	**PLENARY SESSION 6** iPSC Regenerative Medicine				*Plenary Hall*
15:00–15:15	Coffee Break				*Level 5 Foyer*
15:15–16:30	**PLENARY BREAKOUT 1** iPSC and Organogenesis *Room 519AB*	**PLENARY BREAKOUT 2** iPS Generation and Skin and Lung Applications *Room 520CF*	**Q&O TRACK SESSION 7** Regulatory Expectations for the Manufacturing Control Strategy *Room 520BE*	**SFC TRACK SESSION 7** Next Generation Manufacturing for Cell and Gene Therapies *Room 518BC*	

*Quality and Operations Track
** Strategies for Commercialization Track

LEVEL 5
PALAIS DES CONGRÈS

- REGISTRATION
- CONCURRENT SESSIONS
- MEDIA ROOM – 522C
- SPEAKER SERVICES – 521C
- EXHIBIT FUTURE SALES – 521A
- ANCILLARY ROOMS – 521-525
- PARTNERING BOOTHS – 1-12

BOARDROOM 445 – LEVEL 4



International Society
ISCT
Cell & Gene Therapy

IMPROVING MESENCHYMAL STEM CELL POTENCY AND SURVIVAL

Organized by the ISCT MSC Scientific Committee

PRE-CONFERENCE WORKSHOP

MAY 2, 2018 • 8:30 – 16:15
ROOM 520CF

Co-Chairs:
Jan Nolta, *PhD, University of California Davis Stem Cell Program, United States*
Donald G. Phinney, *PhD, The Scripps Research Institute, United States*

SUPPORTERS:

International Society
ISCT
Cell & Gene Therapy

RoosterBio®

Program

08:30–10:00	**SESSION 1 – DEVELOPMENT OF POTENCY ASSAYS TO PREDICT MSC CLINICAL EFFICACY** *Chair:* **Donald G. Phinney,** *PhD, The Scripps Research Institute, United States* *Speakers:* PREDICTING THE CLINICAL EFFICACY OF MSC-BASED THERAPIES **Donald G. Phinney,** *PhD, The Scripps Research Institute, United States* IMPROVING MSC-BASED GENE AND CELL THERAPY **Massimo Dominici,** *MD, University of Modena and Reggio Emilia, Italy* HIGH THROUGHPUT APPROACHES TO ASSESS MSC FUNCTION **Steven R. Bauer,** *PhD, U.S. Food and Drug Administration, United States*
10:00–10:15	Coffee Break
10:15–11:45	**SESSION 2 – METHODS TO IMPROVE MSC ENGRAFTMENT AND POTENCY IN VIVO** *Chair:* **Jan Nolta,** *PhD, University of California Davis Stem Cell Program, United States* *Speakers:* PRE-CONDITIONING OF MSCS TO IMPROVE ENGRAFTMENT AND FUNCTION **Jan Nolta,** *PhD, University of California Davis Stem Cell Program, United States* MSC POTENCY IN SEPTIC SHOCK **David Courtman,** *PhD, The Ottawa Hospital Research Institute, Canada* SCAFFOLDS TO ENHANCE THE REGENERATIVE PROPERTIES OF MESENCHYMAL STROMAL CELLS **Armand Keating,** *MD, University of Toronto, Canada*
11:45–13:00	INDUSTRY LUNCHEON **Jon A. Rowley,** *PhD, RoosterBio Inc., United States*
13:00–14:30	**SESSION 3 – "RESPONDERS" TO MSC THERAPY - PREDICTING CLINICAL OUTCOME** *Chair:* **Katarina Le Blanc,** *MD, PhD, Karolinska Institute, Stockholm, Sweden* *Speakers:* MECHANISTIC INSIGHTS INTO PATIENT RESPONSE RATES FOR MSC-BASED THERAPIES **Katarina Le Blanc,** *MD, PhD, Karolinska Institute, Stockholm, Sweden* ADIPOSE-DERIVED CELLS IN CLINICAL TRIALS OF REGENERATIVE MEDICINE **Eleuterio Lombardo,** *PhD, TiGenix SAU, Spain* MSC-BASED THERAPIES FOR ACUTE RESPIRATORY DISTRESS SYNDROME **Michael Matthay,** *MD, University of California San Francisco, United States* MSC NANOTUBULES AS A CELL-TO-CELL COMMUNICATION SYSTEM IN ACUTE RESPIRATORY DISTRESS SYNDROME **Anna Krasnodembskaya,** *PhD, Queen's University of Belfast, United Kingdom*
14:30–14:45	Coffee Break
14:45–16:15	**SESSION 4 – WRAP UP SUMMARY AND PANEL DISCUSSION** *Chair:* **Jacques Galipeau,** *MD, FRCP(C), University of Wisconsin-Madison, United States* *Speakers:* TRIAL DESIGN: CELL REPLACEMENT OR CELL EMPOWERMENT **Yufang Shi,** *PhD, Soochow University Institutes for Translational Medicine, China* MSCS IN CLINICAL USE – WHERE TO NOW? **Jacques Galipeau,** *MD, FRCP(C), University of Wisconsin-Madison, United States* Q&A SESSION

International Society
ISCT
Cell & Gene Therapy

CELL PROCESSING TRACK

PRE-CONFERENCE WORKSHOP

MAY 2, 2018 • 8:30 – 16:15
ROOM 520BE

Co-Chairs:
David DiGiusto, *PhD, Stanford Healthcare and Stanford School of Medicine, United States*
Ruud Hulspas, *PhD, Cellular Technologies Bioconsulting, United States*
Sarah Nikiforow, *MD, PhD, Dana Farber Cancer Institute, United States*
Denis-Claude Roy, *MD, FRCPC, CellCAN, Maisonneuve-Rosemont Hospital, University of Montréal, Canada*

This track will focus on aspects of cell processing relevant to manufacturing of cell-based products. Experts and Key Opinion Leaders on the topic will share their experiences and views on novel cell processing techniques and cell characterization by flow cytometry. The cell processing track aims at reproducible, safe and sustainable methods applicable in manufacturing settings for cell-based products intended for cellular therapy.

Program

08:30–10:00	**SESSION 1 – NOVEL CELL PROCESSING TECHNOLOGIES** *Chair:* **Sarah Nikiforow,** *MD, PhD, Dana Farber Cancer Institute, United States* *Speakers:* LOCALLY PRODUCED DUAL TARGETED ANTI-CD19/ANTI-CD20 CAR-T CELL THERAPY FOR PATIENTS WITH RELAPSED, REFRACTORY NHL **Nirav Shah,** *MD, MS, Medical College of Wisconsin, United States* IMPROVED CELL RECOVERY AND RESPONSE BY THE USE OF DETERMINISTIC LATERAL DISPLACEMENT IN BLOOD PRODUCT PROCESSING **Tony Ward,** *MBA, GPB Scientific, United States* APPROACHES TO iPSC GENERATION **Vittorio Sebastiano,** *PhD, Stanford School of Medicine, United States*
10:00–10:15	Coffee Break
10:15–11:45	**SESSION 2 – GROWTH AREAS IN NOVEL CELL THERAPY QUALITY CONTROL AND RELEASE TESTING** *Chair:* **David DiGiusto,** *PhD, Stanford Healthcare and Stanford School of Medicine, United States* *Speakers:* APPROACHES TO ASSESSING CELL FUNCTION **Adrian Gee,** *PhD, Baylor College of Medicine, United States* INDUSTRY PERSPECTIVE ON QUALITY CONTROL AND RELEASE TESTING **Jean-Pierre Latere,** *PhD, Celyad, Belgium* VCN AND RCL TESTING FOR LENTIVIRAL VECTORS **Lindsey Skrdlant,** *PhD, Stanford University School of Medicine, United States*
11:45–13:00	Lunch
13:00–14:30	**SESSION 3 – CELL PRODUCT CHARACTERIZATION PART I** *Chair:* **Ruud Hulspas,** *PhD, Cellular Technologies Bioconsulting, United States* *Speakers:* FLOW CYTOMETRY STANDARDIZATION AND ROBUST CELL ANTIGEN QUANTIFICATION: A ROAD TOWARD PREDICTING CELL BIOLOGICAL CHARACTERISTICS **Heba Degheidy,** *MD, PhD, FDA, United States* QUANTITATIVE FLOW CYTOMETRY IN CELL THERAPY – CHALLENGES AND RISK MITIGATION **Ines Mende,** *PhD, Hitachi Chemical Advanced Therapeutic Solutions, LLC* WHEN STEM CELLS MEET IMMUNOTHERAPY: THE CRITICAL ROLE OF MULTI-PARAMETER FLOW CYTOMETRY IN THE DEVELOPMENT OF OFF-THE-SHELF CELL THERAPIES **Tamara Laskowski,** *PhD, MD Anderson Cancer Center, United States*
14:30–14:45	Coffee Break
14:45–16:15	**SESSION 4 – CELL PRODUCT CHARACTERIZATION PART II** *Chair:* **Ruud Hulspas,** *PhD, Cellular Technologies Bioconsulting, United States* *Speakers:* MULTI-PARAMETER FLOW CYTOMETRY IN PRODUCT CHARACTERIZATION OF THERAPEUTIC CELLS **Kathryn Holderness,** *MS, Lonza, United States* MECHANISMS OF RESPONSE AND RESISTANCE TO CD19-TARGETING CHIMERIC ANTIGEN RECEPTORS IN LEUKEMIA **J. Joseph Melenhorst,** *PhD, University of Pennsylvania, United States* CASE STUDIES IN MULTI-PARAMETER FLOW CYTOMETRIC CHARACTERIZATION OF TCR, TIL, AND CAR-T CELL THERAPY PRODUCTS **Sadik Kassim,** *PhD, Mustang Bio, United States*

International Society
ISCT
Cell & Gene Therapy

GRP
GLOBAL REGULATORY PERSPECTIVES

PRE-CONFERENCE WORKSHOP

MAY 2, 2018 • 8:00 – 16:45
ROOM 520AD

Co-Chairs:

Karen Nichols, *Esq., VP, Regulatory and Quality, Magenta Therapeutics, United States*

Dominic Wall, *PhD, FFSc(RCPA), Operations Director, Peter MacCallum Cancer Centre, Australia*

SUPPORTERS:

cell therapies

BURROUGHS WELLCOME FUND

EMO 艾歐生物醫學股份有限公司 EMO BIOMEDICINE CORP.

Educational Grant provided by:

cell therapies

Organizing Committee

CO-CHAIRS:

Karen Nichols, *Esq., VP,* *Regulatory and Quality, Magenta Therapeutics, United States*
Dominic Wall, *PhD, FFSc(RCPA),* *Operations Director, Peter MacCallum Cancer Centre, Australia*

MEMBERS:

Patrick Bedford, *Commercialization Manager, Centre for Commercialization of Regenerative Medicine (CCRM), Canada*
Christopher Bravery, *PhD,* *Director, Consulting on Advanced Biologicals, United Kingdom*
Scott Burger, *MD,* *Principal, Advanced Cell & Gene Therapy, United States*
Srinivasan Kellathur, *PhD,* *Head, Advanced Therapy Products Unit, Health Sciences Authority, Singapore*
Janet Macpherson, *PhD,* *Development Manager, Dept of Cell & Molecular Therapies, Royal Prince Alfred Hospital, Australia*

Program

8:00 – 8:05	**WELCOME** **Karen Nichols,** *Esq., VP, Regulatory and Quality, Magenta Therapeutics, United States* **Dominic Wall,** *PhD, FFSc(RCPA), Operations Director, Peter MacCallum Cancer Centre, Australia*
8:05 – 8:30	**REGULATORY ROUNDUP** *Presenter:* **Scott Burger,** *MD, Principal, Advanced Cell & Gene Therapy, United States*
8:30 – 9:30	**SESSION I: LOCALLY DEVELOPED COMPLEX IMMUNOTHERAPIES FOR GLOBAL SUPPLY** *Moderator:* **Dominic Wall,** *PhD, FFSc(RCPA), Operations Director, Peter MacCallum Cancer Centre, Australia* *Regulator:* **Francesco Cicirello,** *PharmD (GPhC, FOPH), MSc, Inspector, Manufacturing Quality Branch, Medical Devices and Product Quality Division, Therapeutic Goods Administration, Australia (audio conference)* *Industry:* **John Ng,** *BEng, Chief Operations Officer and Executive Director, Tessa Therapeutics Pte Ltd, Singapore*
9:30 – 10:00	**CASE STUDY SESSION I** *Panel Discussion:* **Dominic Wall, Francesco Cicirello, John Ng**
10:00 – 10:15	Coffee Break
10:15 – 11:15	**SESSION II: CMC CONSIDERATIONS WHEN EXPANDING THE MARKET INTO OTHER REGIONS** *Moderator:* **Christopher Bravery,** *PhD, Director, Consulting on Advanced Biologicals, United Kingdom* *Regulator:* **I-Ning Tang,** *MD, MSc, Senior Medical Reviewer, Center for Drug Evaluation (CDE), Taiwan* *Regulator:* **Nadine K. Kolas,** *PhD, Senior Policy Analyst, Health Canada, Canada* *Industry:* **Victor Lietao Li,** *MD, Co-Founder and CEO, Lion TCR Pte Ltd., Singapore*
11:15 – 11:45	**CASE STUDY SESSION II** *Panel Discussion:* **Christopher Bravery, I-Ning Tang, Nadine K. Kolas, Victor Lietao Li**
11:45 – 12:45	Lunch

12:00 – 12:30	**LUNCH SEMINAR PRESENTED BY HEALTH AND ENVIRONMENTAL SCIENCES INSTITUTE (HESI) – CELL THERAPY – TRACKING, CIRCULATION, & SAFETY (CT-TRACS)** **SAFETY ASSESSMENT OF CELL THERAPY PRODUCTS: CHALLENGES AND CURRENT ADVANCES. PERSPECTIVES FROM A NEW INTERNATIONAL MULTI-STAKEHOLDER COLLABORATIVE PLATFORM** *Speakers:* **Brooke M. Helfer,** *PhD, Director of Research and Development, Celsense Inc, United States* **Yoji Sato,** *PhD, Head, Division of Cell-Based Therapeutic Products, National Institute of Health Sciences, Japan*
12:45 – 14:00	**SESSION III: TRIAL DESIGN AND PATIENT COHORTS** *Moderator:* **Karen Nichols,** *Esq., VP, Regulatory and Quality, Magenta Therapeutics, United States* *Regulator:* **Marc Turner,** *MBChB, PhD, MBA, Medical Director, Scottish National Blood Transfusion Service, Scotland* *Regulator:* **Shari Targum,** *MD, MPH, FACC, Chief, General Medicine Branch 1, Division of Clinical Evaluation and Pharmacology/Toxicology, Office of Tissues and Advanced Therapies, Center for Biologics Evaluation and Research, U.S. FDA, United States (audio conference)* *Physician:* **Michael Pepper,** *MBChB, PhD, MD, Director, Institute for Cellular and Molecular Medicine and SAMRC Extramural Unit for Stem Cell Research and Therapy, University of Pretoria, South Africa* *Additional Panelists:* **Steven S. Oh,** *PhD, Deputy Director, Division of Cellular and Gene Therapies, Office of Tissues and Advanced Therapies, Center for Biologics Evaluation and Research, U.S. Food and Drug Administration, United States*
14:00 – 14:30	**CASE STUDY SESSION III** *Panel Discussion:* **Karen Nichols, Marc Turner, Shari Targum, Michel Pepper, Steven S. Oh**
14:30 – 14:45	Coffee Break
14:45 – 15:45	**SESSION IV: BIOLOGICAL VARIATION AND CELL PRODUCT EFFICACY** *Moderator:* **Janet Macpherson,** *PhD, Development Manager, Dept of Cell & Molecular Therapies, Royal Prince Alfred Hospital, Australia* *Regulator:* **Christiane Niederlaender,** *PhD, Senior Quality Assessor , MHRA and CAT-EMA Member, MHRA, United Kingdom* *Regulator:* **Ramjay S. Vatsan,** *PhD, Team Leader, Division of Cellular & Gene Therapies, Office of Tissues and Advanced Therapies, Center for Biologics Evaluation and Research, U.S. FDA, United States* *Industry:* **Erik Rutjens,** *PhD, Global Head, New and Enabling Technologies, Cell and Gene Therapy Development and Manufacturing, Novartis Pharmaceuticals Corporation, United States*
15:45 – 16:30	**CASE STUDY SESSION IV** *Panel Discussion:* **Janet Macpherson, Christiane Niederlaender, Ramjay S. Vatsan, Erik Rutjens, Brooke M. Helfer, Yoji Sato**
16:30 – 16:45	**CLOSING REMARKS**

Session Descriptions

Session I: Locally Developed Complex Immunotherapies for Global Supply

Patient-specific cell-based immunotherapies have evolved into potent therapeutic products that are being developed for multiple markets around the world on the basis of small-scale, single-country clinical studies. When clinical data supporting approval have been generated in a single market, what are the implications for global supply? What considerations arise from the complex interplay of factors such as local donor management and centralized manufacturing located in other jurisdictions, and what does this mean from the perspective of the inspectorates?

Session II: CMC Considerations when Expanding the Market into Other Regions

Autologous cell-based products pose unique challenges because the donor starting material needs to be procured at the hospital and shipped to the manufacturing site within a short period of time (typically less than 48h). Consequently, when expanding operations into other markets, local rules for donor collection must be addressed.

Some new markets will be too far from the initial manufacturing site to allow timely shipment, in which case manufacturing must be located closer to the new market or in it. Even simply replicating the existing process at another site can involve small changes in procedures, equipment, and raw materials; in addition, however, local regulatory expectations may differ.

Session III: Trial Design and Patient Cohorts

Patient-specific factors are fundamental to clinical trial design for any autologous cell therapy. Clinical status, effects of prior and concurrent treatments, cell collection risks, variability of cellular starting material and the manufacturing process, and ethical considerations related to process failures are among the issues that must be addressed.

Session IV: Biological Variation and Cell Product Efficacy

What can be done to predict effectiveness of cell-based immunotherapy?

Inherent variability between products is a given for autologous cellular immunotherapy products due to their patient-derived nature. What strategies can be employed to help predict the likelihood of clinical success of autologous cell products? What do regulators consider important? What should be done to determine the host factors that predict biological response to a product? What technology is available and permitted to assist in determining treatment effectiveness? How much data is required? At what stage of product development should these evaluations be undertaken?

Case Studies

Case Study I

A gene-modified autologous CAR-T cell product has a Biologics License from US FDA for an adult leukemia based upon a B-cell target. The product was developed in the US, has centralized manufacturing in the US, and received FDA approval based on data from US studies and results of FDA inspections.

The company intends to market the product for the same indication in Europe, performing apheresis collections in Europe, shipping to the US manufacturing site, and then shipping the CAR-T cell product back to the European treatment centers.

- What does this mean in terms of inspection of harvesting, manufacturing and release for supply?
- What are the relevant standards, and what happens when they differ? Will the new EU GMP for ATMP have any impacts?
- What is the optimal strategy for the qualified person (QP) supporting external (international) manufacturing?
- What obstacles might be encountered, and how can they be minimized?

The company also plans to market the product for the same indication in an ASEAN country. Apheresis collection and treatment would be performed at clinical sites in Malaysia, and manufacturing would be performed at the US manufacturing site, same as for Europe.

- Questions as for Europe.

The company plans to open a second manufacturing site, to be located in Singapore. Could this site serve as a backup for the US manufacturing site?

Case Study II

It is decided to run two further trials in two different countries. Due to the short shelf-life of the apheresis starting material this requires that the cell manufacturing steps occur locally, although the same vector source (i.e. manufactured in US) will be used. Each manufacturing site will use the same process and the same release criteria as the US-manufactured products. Local differences have been noted in aspects of the apheresis collection (different devices and practices), and largely for logistical reasons some critical raw materials will need to be sourced from different suppliers.

The company plans to undertake a study as part of tech transfer to ensure each new site is comparable to the US-produced product, following ICH Q5E principles. The approach will be to take 3 batches of healthy donor apheresis and split these in two and process one half at each site, and confirm they meet release specifications. Both sites will initiate the batch at the same time to avoid differences in apheresis age. For a nearby country this can be achieved within the apheresis shelf-life, but for a more distant country it is likely the shelf-life would be exceeded.

- Which, if any aspects, of the US product approval are relevant?
  - E.g. will US clinical data be accepted as primary or only supportive?
- Are there procedures to accelerate approval of products with an unmet clinical need?
- Are there specific rules for collecting donor apheresis in your region?
- Are there any issues shipping healthy donor apheresis into your region from a third country (likely US)?
- Is apheresis collection considered part of manufacturing or separate?
- The vector will come from a US FDA licensed GMP facility; will this be acceptable in your region (assuming the quality is suitable)?
- Is ICH Q5E a recognized guideline in your region?
  - Is there any additional local guidance to consider?
- Does the general comparability approach sound suitable?
- Would other studies be required to bridge the locally manufactured product into your market?

Assuming the product was later approved, when making post-approval changes would it be necessary to reconfirm comparability to the US product?

Case Study III

The sponsor decides to adapt the design of the clinical study for adults to a pediatric population in Europe and would like to change the study design as little as possible. Preliminary data suggest that cell populations in starting material collected by apheresis differ between adults and pediatric patients, which may contribute to variability of the manufacturing process, product potency, and potentially, clinical efficacy. Although processing will be centralized, apheresis collections will take place at various clinical sites, and may be modified to accommodate smaller blood volumes of younger patients.

- How should the sponsor manage this variability in the clinical study design?
- What, if any, additional product characterization should be performed on the cellular starting material and the final product. Would this require the addition of new clinical endpoints?

Cell yields from the manufacturing process are expected to vary and will be tracked with in-process controls and release testing.

- To what extent should apheresis collection of starting material be considered part of the trial design, and to what extent are potentially suboptimal cell yields in starting material and/or manufactured product usable in the clinical study? How might inclusion of these products affect the regulatory strategy?

As noted, the leukemia indication for the US approval was for adult patients, and the sponsor wants to initiate one or more pediatric clinical studies to address substantial concerns among European clinicians regarding the exclusion of pediatric patients.

- What are the formal requirements in different agencies for pediatric data to support marketing approval in respective region(s)?
- What is the role of a pediatric investigational plan for treating patients with a personalized therapeutic product?

Given that this is a small (in numbers) patient population, patients who fail to meet inclusion criteria could be a substantial percentage of the total patient population.

- Should the sponsor opt for expanded access for these patients? What is the regulatory view of doing so for a CAR-T cell therapy product?

Case Study IV

It becomes evident that some patients have a very poor response to this cellular immunotherapy product, leading one investigator to suggest that the poor outcomes are seen predominantly in patients with leukemic cells in the CNS. However, pre-clinical and earlier clinical studies suggested that CNS disease would be controlled by the immunotherapy, although CNS-specific clinical data and samples were not collected or preserved in a standardized and robust manner, e.g., some data points are missing or uninterpretable due to lack of standardization in the assessments.

- How valuable is biodistribution data for cell-based therapies?
- What time frame is likely to provide the most robust data?
- Do regulators ever ask for clinical pharmacokinetic data for cell therapy products? If so, what circumstances would prompt such a request?
- How relevant might animal models be in predicting distribution of the cells in humans?
- What imaging strategies can be considered that support the proof of concept for the mode of action without compromising product quality?
- If an imaging cohort is planned, how experimental can the imaging method and labels be?
- How could comparability of the product before and after labeling be assessed?
- Should studies have different patient cohorts to facilitate imaging with more evident targets?

International Society
ISCT
Cell & Gene Therapy

ISCT-FACT CELL THERAPY QUALITY BOOT CAMP

PRE-CONFERENCE WORKSHOP

MAY 2, 2018 • 8:30 – 16:15
ROOM 519AB

SUPPORTERS:

International Society
ISCT
Cell & Gene Therapy

fact
FOUNDATION FOR THE ACCREDITATION OF CELLULAR THERAPY
AT THE UNIVERSITY OF NEBRASKA MEDICAL CENTER

Program

08:30 – 10:00	**SESSION 1 – QUALITY MANAGEMENT PROGRAM** ADAPTING A QUALITY MANAGEMENT PROGRAM TO FIT YOUR NEEDS **Natasha Lapteva,** ***PhD,*** *Houston Methodist Hospital Bone Marrow Transplant (affiliated with Baylor College of Medicine Cell and Gene Therapy), United States* QUALITY MANAGEMENT ROUNDTABLE
10:00 – 10:15	Coffee Break
10:15 – 11:45	**SESSION 2 – VALIDATION, VERIFICATION, AND QUALIFICATION** USING VALIDATION, VERIFICATION, AND QUALIFICATION TO ENSURE PROPER EQUIPMENT OPERATION **Nancy Collins,** ***PhD,*** *FACT Consultant and Clinical Professor at the University of Toledo, United States* **Deborah Griffin,** ***MSc, ASQ CPGP,*** *FACT Consultant, United States* VALIDATION, VERIFICATION, AND QUALIFICATION ROUNDTABLE
11:45 – 13:00	Lunch
13:00 – 14:30	**SESSION 3 – AUDITING** CLOSING THE AUDIT PROCESS THROUGH FOLLOW-UP **Nicole Prokopishyn,** ***PhD,*** *Alberta Blood and Marrow Transplant Program: Foothills Medical Centre, Alberta Children's Hospital, Tom Baker Cancer Centre, and Calgary Laboratory Services, Canada* AUDIT ROUNDTABLE
14:30 – 14:45	Coffee Break
14:45 – 16:15	**SESSION 4 – OCCURRENCE MANAGEMENT** GOING BEYOND OCCURRENCE MANAGEMENT TO MITIGATE RISKS **Mary Ann Kelley,** ***MT(ASCP)BB,*** *Dana-Farber Cancer Institute / Cell Manipulation Core Facility, United States* OCCURRENCE MANAGEMENT ROUNDTABLE

International Society
ISCT
Cell & Gene Therapy

ISCT-CBA CORD BLOOD SERIES

IN PARTNERSHIP WITH ASBMT

PRE-CONFERENCE WORKSHOP

MAY 2, 2018 • 8:30 – 16:15
ROOM 518A

Co-Chairs:
Joanne Kurtzberg, *MD, Carolinas Cord Blood Bank at Duke University Medical Center, United States*
Elizabeth J. Shpall, *MD, MD Anderson Cancer Center, United States*

SUPPORTERS:

International Society
ISCT
Cell & Gene Therapy

ASBMT
American Society for Blood and Marrow Transplantation

CORD BLOOD ASSOCIATION

Program

08:30 – 10:00	**SESSION 1 – CURRENT STATUS OF CORD BLOOD TRANSPLANTATION** *Speakers:* LESSONS LEARNED FROM CORD BLOOD BANKING AND TRANSPLANTATION **Joanne Kurtzberg,** *MD, Carolinas Cord Blood Bank at Duke University Medical Center, United States* EXPANSION AND HOMING **Elizabeth J. Shpall,** *MD, MD Anderson Cancer Center, United States* CLINICAL EXPERIENCE WITH A NOVEL CORD BLOOD EXPANSION TECHNOLOGY **Mitchell Horwitz,** *MD, Duke University School of Medicine, United States*
10:00 – 10:15	Coffee Break
10:15 – 11:45	**SESSION 2 – CORD BLOOD IMMUNOTHERAPY** *Speakers:* CORD BLOOD NK CELLS **Katy Rezvani,** *MD, PhD, MD Anderson Cancer Center, United States* CORD BLOOD T CELLS (INCLUDING HIV) **Catherine Bollard,** *MBChB, MD, Children's National Medical Center and The George Washington University, United States* NEW CBT TRIALS INVESTIGATING THIRD PARTY PROGENITORS AND EC BASED EXPANSION **Juliet Barker,** *MBBS, FRACP, Memorial Sloan Kettering Cancer Center, United States*
11:45 – 13:00	Lunch
13:00 - 14:30	**SESSION 3 – MANUFACTURING NOVEL CELL THERAPY PRODUCTS FROM CORD AND BLOOD TISSUE** *Speakers:* CHARACTERIZATION OF MSCS DERIVED FROM VARIOUS DONOR SOURCES **Carolyn Yeago,** *PhD, Georgia Institute of Technology, United States* MANUFACTURING OF CORD TISSUE MSCS **Joanne Kurtzberg,** *MD, Carolinas Cord Blood Bank at Duke University Medical Center, United States* TREATMENT OF PATIENTS WITH FRAILTY SYNDROMES WITH MSCS **Ivonne Hernandez Schulman,** *MD, University of Miami Miller School of Medicine, United States* CORD TISSUE MSC-DERIVED EXOSOMES **Elizabeth J. Shpall,** *MD, MD Anderson Cancer Center, United States* MSC THERAPY FOR STEROID REFRACTORY GVHD IN PEDIATRIC PATIENTS **Joanne Kurtzberg,** *MD, Carolinas Cord Blood Bank at Duke University Medical Center, United States*
14:30 – 14:45	Coffee Break
14:45 – 16:15	**SESSION 4 – CORD BLOOD FOR REGENERATIVE MEDICINE** *Speakers:* OUTCOMES OF CORD BLOOD THERAPIES IN PEDIATRIC NEUROLOGIC DISEASES **Joanne Kurtzberg,** *MD, Carolinas Cord Blood Bank at Duke University Medical Center, United States* MSC TREATMENT OF CHRONIC PULMONARY DISEASES IN ADULTS **Marilyn Glassberg,** *MD, University of Miami Miller School of Medicine, United States* MSCS – NOVEL THERAPY FOR PATIENTS WITH CYSTIC FIBROSIS **Arnold Caplan,** *PhD, Case Western Reserve University, United States*

International Society
ISCT
Cell & Gene Therapy

CANADIAN CELL & GENE THERAPY STRATEGY WORKSHOP

PRE-CONFERENCE WORKSHOP

MAY 2, 2018 • 8:30 – 16:15
ROOM 518BC

Co-Chairs:
Denis-Claude Roy, *MD, FRCPC, Chief Executive Officer, CellCAN, Canada*
Craig Hasilo, *Chief Scientific Officer, CellCAN, Canada*

SUPPORTERS:

International Society
ISCT
Cell & Gene Therapy

CellCAN
Regenerative Medicine and
Cell Therapy Network

Program

Workshop Moderator:

Michel Rochon, *Health and Science Journalist, Broadcaster, Lecturer, Montréal, Canada*

Organizing Committee:

Renée Bazin, *Directeur, Innovation, Affaires Médicales et Innovation, Héma-Québec*
Patrick Bedford, *Senior Manager, Clinical Translation & Regulatory Affairs, Centre for Commercialization of Regenerative Medicine*
Sandra Donaldson, *Vice President & Chief Operating Officer, Ontario Institute for Regenerative Medicine*
Julie Fradette, *Directrice, ThéCell*
Jodi Garner, *Senior Manager, Science and Industry Relations, Ontario Institute for Regenerative Medicine*
Vanessa Laflamme, *Chief Operating Officer, CellCAN Regenerative Medicine and Cell Therapy Network*
Amanda MacDonald, *Executive Director, BC RegMed*
Stephanie Michaud, *President & Chief Executive Officer, BioCANRx*
Cate Murray, *Executive Director & Chief Operating Officer, Stem Cell Network*
Louisa Petropoulos, *Director, Business Development and Operations, Centre for Commercialisation of Cancer Immunotherapy (C3i)*
Friedericke Pfau, *Chargée de projets LOEX, CMDGT*
Michel Tremblay, *James McGill Professor, McGill University*
Frank Van Lier, *Director R&D, Bioprocess Engineering, Life Sciences – Human Health Therapeutics, National Research Council Canada*

08:30–10:00	**SESSION 1 – CELL & GENE THERAPY MANUFACTURING INFRASTRUCTURE** ***Chair:*** **Armand Keating,** ***MD,*** *Director, Division of Hematology, Epstein Chair in Cell Therapy and Transplantation, University of Toronto, Canada* ***Speakers:*** CURRENT STATE OF GMP MANUFACTURING IN CANADA **Denis-Claude Roy,** ***MD, FRCPC,*** *CEO, CellCAN, Montréal, Canada* CURRENT STATE OF GMP MANUFACTURING IN FRANCE – BIOREGATE PERSPECTIVE **Oumeya Adjali,** ***MD, PhD,*** *Directrice du laboratoire de thérapie génique de Nantes, Unité de thérapie cellulaire et génique du CHU de Nantes, Nantes, France* CURRENT STATE OF GMP MANUFACTURING IN AUSTRALIA **John Rasko,** ***MD, PhD,*** *ISCT President-Elect, Professor of Medicine, Central Clinical School, Centenary Institute of Cancer Medicine & Cell Biology, Sydney, Australia* MANUFACTURING REQUIREMENTS FOR CAR-T CELL THERAPIES AND ENGINEERED CELLS **Isabelle Rivière,** ***PhD,*** *Director, Cell Therapy and Cell Engineering Facility, Memorial Sloan Kettering Cancer Centre, New York, United States*
10:00–10:15	Coffee Break
10:15–11:45	**SESSION 2 – INVESTMENT IN CANADA: THE GLOBAL ADVANTAGE** ***Chair:*** **Michael May,** ***PhD,*** *President & Chief Executive Officer, Centre for Commercialization of Regenerative Medicine, Toronto, Canada* ***Speakers:*** LEVERAGING FUNDING FOR GMP MANUFACTURING INFRASTRUCTURE – FEDERAL SUPPORT OF INDUSTRY INVESTMENT: WHAT MORE BANG FOR YOUR BUCK GETS YOU **Aaron Dulgar-Tulloch,** ***PhD,*** *Director, BridGE, GE Healthcare, Toronto, Canada* DOING BUSINESS IN CANADA – DEVELOPING BEST IN CLASS IPSC THERAPIES AS AN INDUSTRY LEADER **Robert Deans,** ***PhD,*** *Chief Technology Officer, BlueRock Therapeutics, Toronto, Canada* BIOMANUFACTURING FOR CELL AND GENE THERAPY PRODUCTION IN CANADA **Frank Van Lier,** ***PhD,*** *Director R&D, Bioprocess Engineering, Life Sciences, Human Health Therapeutics, National Research Council Canada, Montréal, Canada*

11:45–13:00	Lunch
13:00–14:30	**SESSION 3 – MAKING FRONTLINE LIFE SAVING THERAPIES ACCESSIBLE & ECONOMICALLY FEASIBLE** ***Chair:*** **Lambert Busque,** ***MD,*** *Chief Medical Officer, Center for Commercialisation of Cancer Immunotherapy, Montréal, Canada* ***Speakers:*** HEALTH CANADA'S NOC/C: REQUIREMENTS FOR ACCESSING NOVEL FRONTLINE LIFESAVING CELL & CELL-BASED GENE THERAPIES **Kelly Robinson,** ***MSc,*** *Director, Centre for Evaluation of Radiopharmaceuticals & Biotherapeutics, Biologics and Genetic Therapies Directorate, Health Canada, Ottawa, Canada* THE CHALLENGES TO HTA AGENCIES OF EXPEDITED REGULATORY APPROVALS AND APPROACHES TO MANAGING THE CONSEQUENT RISK **Nick Crabb,** ***PhD,*** *Programme Director, Scientific Affairs, National Institute for Health and Care Excellence, London, United Kingdom* RISK SHARING MODELS FOR REIMBURSEMENT **Richard Maziarz,** ***MD,*** *Professor of Medicine, Oregon Health & Science University, Chair, Interest Group for ASBMT on Value and Health Economics, Portland, United States*
14:30–14:45	Coffee Break
14:45–16:15	**SESSION 4 – STRENGTHENING THE CANADIAN RMCT ECOSYSTEM** ***Speakers:*** THE REGENERATIVE MEDICINE ALLIANCE OF CANADA **Michael Rudnicki,** ***PhD,*** *Chair, Regenerative Medicine Alliance of Canada, Scientific Director, Stem Cell Network, Ottawa, Canada* EXAMINING CANADA'S CLINICAL TRIALS ENVIRONMENT **Erika Kleiderman,** ***BSc, LLB,*** *Academic Associate, Centre of Genomics and Policy, McGill University, Montréal, Canada* SUCCESSFUL CANADIAN THERAPEUTICS MANUFACTURING: CHALLENGES OF SCALE-UP AND EXPANSION **Dina Iezzi,** *Director, Marketing & Special Projects, Therapure Biopharma Inc, Toronto, Canada* BUILDING SUCCESSFUL INTERNATIONAL COLLABORATIONS **Duncan Stewart,** ***MD,*** *President and Scientific Director, Ontario Institute for Regenerative Medicine, Ottawa, Canada*

Notes

Alireza Abazari, *MSc, PhD, BioLife Solutions, United States*
Julie Allickson, *PhD, Wake Forest Institute for Regenerative Medicine, United States*
Harold Atkins, *MD, FRCPC, The Ottawa Hospital and Ottawa Hospital Research Institute, Canada*
Christina Bachmeier, *PharmD, BCOP, Moffitt Cancer Center, United States*
Juliet Barker, *MBBS, FRACP, Memorial Sloan Kettering Cancer Center, United States*
John Barrett, *MD, George Washington University Cancer Center, United States*
Shirley Bartido, *PhD, MBA, Cellectis, United States*
Gerhard Bauer, *PhD, University of California Davis, United States*
Rosemarie Bell, *BAppSc Micro/Biochem MASM, QIMR Berghofer Medical Research Institute, Australia*
John Bell, *PhD, The Ottawa Hospital Research Institute, Canada*
Gilbert Bernier, *PhD, University of Montréal, Canada*
Alice Bertaina, *MD, PhD, Stanford University, United States*
Valkal Bhatt, *PharmD, Memorial Sloan Kettering Cancer Center, United States*
James Blackwell, *PhD, MBA, MS, The Windshire Group, LLC, United States*
Catherine Bollard, *MBChB, MD, Children's National Medical Center and The George Washington University, United States*
Alan Burns, *PhD, Takeda Pharmaceuticals, United States*
Stephan Busque, *MD, MSc, Stanford University, United States*
Jonathan Campbell, *PhD, LGC, United Kingdom*
Chulhee Choi, *MD, PhD, KAIST, Republic of Korea*
Rachele Ciccocioppo, *MD, University of Verona, Italy*
Dominic Clarke, *PhD, Charter Medical, United States*
Tammy Clifford, *PhD, Canadian Agency for Drugs and Technologies in Health (CADTH), Canada*
Sarah Cooley, *MD, University of Minnesota Department of Medicine, United States*
Megan Cornelison, *MS, PA-C, MD Anderson Cancer Center, United States*
Randolph Corteling, *PhD, ReNeuron, United Kingdom*
Colleen Delaney, *MD, MSc, Nohla Therapeutics, United States*
Alex Denoon, *BSc, LLB, Marriott Harrison UK, United Kingdom*
David DiGiusto, *PhD, Stanford Healthcare and Stanford School of Medicine, United States*
Massimo Dominici, *MD, University of Modena and Reggio Emilia, Italy*
Dawn Driscoll, *MBA, PhD, Cell Therapies PTY, Australia*
Matthew Durdy, *MBA, FCSI, Cell and Gene Therapy Catapult, United Kingdom*
Shannon Eaker, *PhD, General Electric – GE, United States*
Paul Eldridge, *PhD, UNC Lineberger Advanced Cellular Therapeutics Facility, United States*
Simon Ellison, *MBA, World Courier, United Kingdom*
Solveig Ericson, *MD, PhD, Novartis, United States*
Steven Fischkoff, *MD, WindMIL Therapeutics, United States*
Tobi Fisher, *MPAS, PA-C, MD Anderson Cancer Center, United States*
Flagg Flanagan, *OPM, DiscGenics, Inc., United States*
Miguel Forte, *MD, PhD, Zelluna Immunotherapy, Belgium*
Jacques Galipeau, *MD, University of Wisconsin-Madison, United States*
Heather Garrity, *MHA, Dana Farber Cancer Institute, United States*
Lucie Germain, *PhD, Université Laval and CHU de Québec, Canada*
Rebecca Gonzalez, *PharmD, BCOP, Moffitt Cancer Center, United States*
May Griffith, *PhD, Maisonneuve-Rosemont Hospital Research Centre, University of Montréal, Canada*
Bambi Grilley, *RPH, RAC, CIP, CCRC, CCRP, Baylor College of Medicine, United States*
Patrick Hanley, *PhD, Children's National Health System, United States*
Marie-Josée Hébert, *MD, FRCPC, University of Montréal, Canada*
Uri Herzberg, *DVM, PhD, MBA, Celularity, United States*
Helen Heslop, *MD, Baylor College of Medicine, United States*
Jöns Hilborn, *PhD, Uppsala University, Sweden*
Michael Holmes, *PhD, Sangamo Therapeutics, Inc., United States*
Martin Hoogduijn, *PhD, Erasmus MC, Netherlands*
Karin Hoogendoorn, *PharmD, Leiden University Medical Centre, The Netherlands*
George Hucks, *MD, University of North Carolina Hospitals, United States*
Eric Isberg, *Entegris, United States*
Carl June, *MD, University of Pennsylvania, United States*
Ohad Karnieli, *PhD, MBA, Atvio Biotech, Israel*
Sadik Kassim, *PhD, Mustang Bio, United States*
Armand Keating, *MD, University of Toronto, Canada*
Steven Keizer, *BSc, MBA, CCRM, Canada*
Aisha Khan, *MSc, MBA, University of Miami, United States*
Timothy Kieffer, *PhD, University of British Columbia, Canada*
Sven Kili, *MD, GSK Gene Therapy, United Kingdom*
Donald Kohn, *MD, University of California, Los Angeles, United States*
Nadine Kolas, *PhD, Health Canada, Canada*
Lambros Kordelas, *MD, University Hospital Essen, Germany*
Joanne Kurtzberg, *MD, Carolinas Cord Blood Bank at Duke University Medical Center, United States*
Didier Leconte, *MBA, ASC, Fonds de solidarité FTQ, Canada*
Joseph Leventhal, *MD, PhD, Northwestern University, United States*
Aaron Levine, *PhD, MPhil, School of Public Policy, Georgia Tech, United States*
Sai-Kiang Lim, *PhD, Institute of Medical Biology (IMB) Agency for Science and Technology, (A*STAR), Singapore*
Bangon Longsomboon, *MA, Interdisciplinary Stem Cell Institute, Miller School of Medicine, University of Miami, United States*
Monica Luchi, *MD, FACR, MBA, Celularity, Inc. United States*
Crystal Mackall, *MD, Stanford University, United States*
Nadim Mahmud, *MD, PhD, University of Illinois Hospital and Health Sciences System, United States*
Rocio Manghani, *MPH, Kite, A Gilead Company, United States*
Linda Marban, *PhD, Capricor Therapeutics, United States*
Robert Margolin, *MBA, TrakCel, United States*
Peter Marks, *MD, PhD, US Food and Drug Administration, United States*
Rainer Marksteiner, *PhD, Innovacell Biotechnologie AG, Austria*
John Martin, *BA LLB (Hons), Regeneus Ltd, Australia*
Fernanda Masri, *PhD, Sartorius-Stedim Biotech, United Kingdom*
Michael Matthay, *MD, University of California San Francisco, United States*
Robert W. Mays, *PhD, Athersys, United States*
Gerry McKiernan, *BSc, Cell Therapies PTY Ltd, Australia*
Michael Mendicino, *PhD, Hybrid Concepts International, United States*
Everett Meyer, *MD, PhD, Stanford Healthcare and Stanford University, United States*
Kenneth Micklethwaite, *MBBS, PhD, FRACP, FRCPA, Westmead Hospital, Australia*
William Milligan, *BSc, Steminent Biotherapeutics Inc, Canada/Taiwan/United States*
Maria Mirotsou, *PhD, Astellas Institute of Regenerative Medicine, United States*
Keith Moore, *DC, Canada*
Julie Murrell, *PhD, MilliporeSigma, United States*
Andras Nagy, *PhD, Lunenfeld-Tanenbaum Research Institute, Sinai Health System, Canada*
Robert Negrin, *MD, Stanford University, United States*
Sarah Nikiforow, *MD, PhD, Dana Farber Cancer Institute, United States*
Colin Novick, *CJ Partners, Japan*
Daniel Ollendorf, *PhD, Institute for Clinical and Economic Review (ICER), United States*
Douglas Olson, *PhD, Tekjo Consulting, United States*

Gabrielle O'Sullivan, *PhD, MPH, Royal Prince Alfred Hospital, Australia*
Diane Parks, *MBA, Kite, A Gilead Company, United States*
Bruno Péault, *PhD, University of Edinburgh, Scotland, and University of California at Los Angeles, United States*
Gary Pigeau, *PhD, GE Healthcare, Canada*
Matthew Porteus, *MD, PhD, Stanford University School of Medicine, United States*
Robert Preti, *PhD, Hitachi Chemical Advanced Therapeutics Solutions, United States*
Shahin Rafii, *MD, Weill Cornell Medical College, United States*
Qasim Rafiq, *PhD, University College London, United Kingdom*
Mahendra Rao, *MBBS, PhD, Mahendra Rao LLC, United States*
John Rasko, *AO, BSc(Med), MBBS(Hons), PhD, MAICD, FFSc(RCPA), FRCPA, FRACP, FAHMS, Royal Prince Alfred Hospital, Australia*
Katy Rezvani, *MD, PhD, The University of Texas MD Anderson Cancer Center, United States*
Stanley Riddell, *MD, Fred Hutchinson Cancer Research Center, United States*
Patrick Rivers, *BSc, MBA, Aquilo Capital, United States*
Isabelle Rivière, *PhD, Memorial Sloan Kettering Cancer Center, United States*
Eva Rohde, *MD, Paracelsus Medical University Salzburg, Austria*
Christine Rosati, *Dana Farber Cancer Institute/Boston's Children's Hospital, United States*
Jon Rowley, *PhD, RoosterBio, United States*
Denis-Claude Roy, *MD, FRCPC, CellCAN, Maisonneuve-Rosemont Hospital, University of Montréal, Canada*
Gregory Russotti, *PhD, Celgene Corp, United States*
Paula Salmikangas, *PhD, Adj. Prof, NDA Advisory Services Limited, United Kingdom*
Guy Sauvageau, *MD, PhD, IRIC - Université de Montréal, Canada*
Andrew Scharenberg, *MD, Casebia Therapeutics, United States*
Joseph Schwartz, *MD, MPH, Columbia University Medical Center, United States*
Randolph (Randy) Schweickart, *MS, Juno Therapeutics, United States*
Khalid Shah, *MS, PhD, Harvard Medical School, United States*
Judith Shizuru, *MD, PhD, Stanford University Medical Center, United States*
Daniel Shoemaker, *PhD, Fate Therapeutics, United States*
Julianne Smith, *PhD, Cellectis, France*
Sandeep Soni, *MD, Stanford University, Lucile Packard Children's Hospital, United States*
Elena Spanjaard, *PhD, Pfizer, United States*
Karl Stasko, *MPH, Dana Farber Cancer Institute, Cell Manipulation Core Facility, United States*
Duncan Stewart, *MD, The Ottawa Hospital Research Institute, Canada*
Christopher Storbeck, *PhD, BGTD, Health Canada, Canada*
Jean Tang, *MD, PhD, Stanford University, United States*
Bernard Thébaud, *MD, PhD, The Ottawa Hospital Research Institute, Canada*
Pascal Touchon, *DVM, MBA, Novartis, United States*
Chy-Anh Tran, *MBA, Stanford University, United States*
Eve Tsai, *MD, PhD, FRCSC, The Ottawa Hospital, Ottawa Hospital Research Institute, University of Ottawa, Canada*
Zlatibor Velickovic, *PhD, Royal Prince Alfred Hospital, Australia*
Gabor Veres, *PhD, bluebird bio, United States*
Sowmya Viswanathan, *PhD, University Health Network, University of Toronto, Canada*
Daniel J. Weiss, *MD, PhD, University of Vermont School of Medicine, United States*
Yutaka Yanagita, *PhD, Astellas Pharma Inc., Japan*
Christina Yi, *BS, Dendreon, United States*
John Zaia, *MD, Beckman Research Institute of City of Hope, United States*
Jiwen Zhang, *PhD, The Standards Coordinating Body, United States*
Claudia Zylberberg, *PhD, Akron Biotech, United States*

Oral Abstract Presenters

Holly Anderson, *BSc, Dana Farber Cancer Institute, United States*
Houria Bachtarzi, *PhD, ERA Consulting (UK) Ltd., United Kingdom*
Cynthia Bamdad, *PhD, Minerva Biotechnologies, United States*
Renée Bazin, *PhD, Héma-Québec, Canada*
Ksenia Bezverbnaya, *BSc, McMaster University, Canada*
David Bishop, *MBBS, Westmead Institute for Medical Research, Australia*
Yelena Boccacci, *BSc, Université Laval, Héma-Québec, Canada*
Jaap Jan Boelens, *MD, PhD, UMC Utrecht, Netherlands*
Rachel Burga, *BSc, George Washington University, United States*
Anastasia Cheng, *MSc, McGill University, Canada*
Shant Der Sarkissian, *PhD, MBA, Université de Montréal/CRCHUM, Canada*
Vincenzo Di Cerbo, *PhD, Cell and Gene Therapy Catapult, United Kingdom*
Alla Dolnikov, *PhD, Sydney Children's Hospital, Australia*
Ngaire Elwood, *PhD, BMDI Cord Blood Bank, Australia*
Ahmad Galuta, *Ottawa Hospital Research Institute, Canada*
Sabiha Hacibekiroglu, *PhD, Lunenfeld-Tanenbaum Research Institute/Mt Sinai Health System*
Christopher Helsen, *PhD, Triumvira, Canada*
Mirabelle Ho, *PhD, Ottawa Hospital Research Institute, Canada*
Miriel Ho, *PhD, Ottawa Hospital Research Institute, Canada*
Alexander Johnston, *PhD, CReATe Fertility Centre, Canada*
Ewelina Kurtys, *PhD, King's College London, United Kingdom*
Shirin Lak, *PhD, Maisonneuve Rosemont Hospital, Canada*
William Lemieux, *MSc, CHU Sainte-Justine, Canada*
Rebecca Lim, *PhD, Hudson Institute of Medical Research, Australia*
Roberta Mazzieri, *PhD, University of Queensland, Australia*
Jennifer Moseley, *PhD, Capricor Therapeutics, United States*
Gustavo Moviglia, *MD, Maimonides University, Argentina*
Kai Nesemann, *PhD, Sartorius Lab Instruments GmbH, Germany*
Sarah Nikiforow, *MD, PhD, Dana Farber Cancer Institute, United States*
Robert Nordon, *MBBS, PhD, University of New South Wales, Australia*
Dani Offen, *PhD, Tel Aviv University, Israel*
Amanda Olson, *MD, The University of Texas MD Anderson Cancer Center, United States*
Jeremiah Oyer, *BS, University of Central Florida, United States*
Ioannis Papantoniou, *PhD, KU Leven, Belgium*
Shabnum Patel, *PhD, The George Washington University, United States*
Claudia Raggi, *MD, CHU Sainte-Justine, Canada*
Denis-Claude Roy, *MD, FRCPC, CellCAN, Maisonneuve-Rosemont Hospital, University of Montréal, Canada*
Joel Simpson-Edin, *MSc, Uppsala University, Sweden*
Yashbir Singh, *Chung Yuan Christian University, Taiwan*
Christian Tebid, *MSc, crHMR-UdeM, Canada*
Wei Seong Toh, *National University of Singapore, Singapore*
Sowmya Viswanathan, *PhD, University Health Network, University of Toronto, Canada*

Program Schedule

Wednesday, May 2

	07:00–20:30	Registration	*Level 5 Foyer*
	07:00–18:00	Speaker Services	*Room 521C*
Concurrent Sessions	08:00–16:45	**ISCT GLOBAL REGULATORY PERSPECTIVES (GRP) WORKSHOP***	*Room 520 AD*
Concurrent Sessions	08:30–16:15	**MSC WORKSHOP ON POTENCY ASSAYS***	*Room 520CF*
Concurrent Sessions	08:30–16:15	**ISCT-CBA CORD BLOOD SERIES IN PARTNERSHIP WITH ASBMT***	*Room 518A*
Concurrent Sessions	08:30–16:15	**ISCT CELL PROCESSING TRACK***	*Room 520BE*
Concurrent Sessions	08:30–16:15	**ISCT-FACT CELL THERAPY QUALITY BOOTCAMP***	*Room 519AB*
Concurrent Sessions	08:30–16:15	**CANADIAN CELL & GENE THERAPY STRATEGY WORKSHOP***	*Room 518BC*
	16:30–18:30	**CORPORATE SYMPOSIUM HOSTED BY MILTENYI BIOTEC** **Emerging Technologies and Clinical Advancements in Cell Therapy**	*Room 520CF*
	19:00–19:30	**PRESIDENT'S WELCOME ADDRESS**	*Plenary Hall*
	19:30–21:30	**ISCT 2018 WELCOME RECEPTION AND EXHIBIT OPEN HOUSE**	*Exhibit Hall*

**Pre-Conference Day Registration Required*

Thursday, May 3

	07:00–19:00	Registration	*Level 5 Foyer*
	07:00–18:00	Speaker Services	*Room 521C*
Concurrent Sessions	07:30–08:30	**HOT TOPIC SESSION 1 – Tregs and Other Cell Subtypes/Immunotherapy in Autoimmune Disease** *0.5 AMA PRA Category 1 Credits™* ***Chair: David DiGiusto** (US)* ***Speakers:*** **Everett Meyer** *(US)* **Alan Burns** *(US)* – The Emerging Role of Cell Therapies in Gastrointestinal Disorders	*Room 520BE*
Concurrent Sessions	07:30–08:30	**HOT TOPIC SESSION 2 – Approaches to Overcoming Limitations of Cell and Gene Therapies** *1.0 AMA PRA Category 1 Credits™* ***Chair: Sandeep Soni** (US)* ***Speakers:*** **Gabor Veres** *(US)* – Transduction Efficience is a Critical Component Defining the Clinical Outcome in Gene Therapy **Judith Shizuru** *(US)* – Antibody-based Conditioning for Hematopoeitic Stem Cell Therapies **Daniel Shoemaker** *(US)* – Programmed Cellular Immunotherapies for Fighting Cancer	*Room 520CF*
Concurrent Sessions	07:30–08:30	**CORPORATE BREAKFAST TUTORIAL HOSTED BY GE HEALTHCARE** **Learning by Doing: Learn from Clinical and Manufacturing Leaders' Through Their Journey from Development to Delivery of Cellular Therapies**	*Room 518BC*
Concurrent Sessions	07:30–08:30	**CORPORATE BREAKFAST TUTORIAL HOSTED BY ROCHE DIAGNOSTICS** **Rapid Microbiological Methods in Meeting Unique QC and Product-Specific Validation Needs of Advanced Therapy Manufacturing**	*Room 519AB*

Thursday, May 3

08:45–09:00	**OPENING REMARKS AND PRESENTATION OF 2018 CAREER ACHIEVEMENT AWARD IN CELLULAR THERAPY TO NANCY H. COLLINS, PhD**	*Plenary Hall*
09:00–10:30	**Presidential Plenary – Cancer Immunotherapy** *1.5 AMA PRA Category 1 Credits™* ***Chair: Catherine Bollard (US)*** ***Speakers:*** **Stanley Riddell** *(US)* – Strategies for Improving Efficacy and Safety of CAR T-Cells for Cancer Therapy **Crystal Mackall** *(US)* – CAR T-cells for Cancer: Progress and Challenges **Robert Negrin** *(US)* – Allogeneic Cells with Limited GVHD Potential for Cancer Immunotherapy	*Plenary Hall*
10:30–11:00	Coffee Break with Exhibits	*Exhibit Hall*
10:30–10:45	**CORPORATE PRODUCT THEATRE HOSTED BY GE HEALTHCARE** **Simplify Upstream and Downstream Cell Therapy Processing with GE's Sefia™ S-2000 System**	*Global Product Showcase (Exhibit Hall)*
10:45–11:00	**CORPORATE PRODUCT THEATRE HOSTED BY MILLIPORESIGMA** **Expansion of Human Induced Pluripotent Stem Cells in Suspension Culture**	*Global Product Showcase (Exhibit Hall)*
11:00–12:15	**PLENARY BREAKOUT 1 – Cancer Immunotherapy I** ***Chair: Crystal Mackall** (US)* **ORAL ABSTRACT PRESENTATIONS:** **Cynthia Bamdad** *(US)* – Novel CAR T that Targets MUC1* Not Full-Length MUC1 for Treatment of Solid Tumor Cancers (Abstract 1) **Rachel Burga** *(US)* – Engineering the TGFβ Receptor to Enhance the Therapeutic Potential of Natural Killer Cells as an Immunotherapy for Neuroblastoma (Abstract 2) **Alla Dolnikov** *(AU)* – Factors Promoting CD19-Negative Relapses Following CAR19T Cell Therapy (Abstract 3) **Christopher Helsen** *(CA)* – T-Cells Engineered with T-Cell Antigen Coupler (TAC) Receptors Display Robust Efficacy Against Solid and Liquid Tumor Xenografts in the Absence of Any Toxicity (Abstract 4) **William Lemieux** *(CA)* – Simultaneous Dual CAR Expression to Prevent Relapse in Pre-B Acute Lymphoblastic Leukemia (Abstract 5)	*Room 519AB*
	PLENARY BREAKOUT 2 – Cancer Immunotherapy II ***Chair: Robert Negrin** (US)* **ORAL ABSTRACT PRESENTATIONS:** **Ksenia Bezverbnaya** *(CA)* – BCMA-Specific TAC Receptor-Engineered T Cells For Multiple Myeloma (Abstract 6) **David Bishop** *(AU)* – shRNA-Mediated TCR Knockdown as a Foundation for Allogeneic CAR19 T-Cells Generated by Single-Step Genetic Modification with the piggyBac Transposase (Abstract 7) **Shirin Lak** *(CA)* – Modulation of Co-Signaling for Improved Ex Vivo Human Antigen-Specific T-Cells Generation For Immunotherapy (Abstract 8) **Shabnum Patel** *(US)* – HIV-Specific T-Cells can be Generated Against Conserved Non-Escaped HIV Epitopes for Use in a Phase I Clinical Trial: Pre-Clinical Validations and Implications for a Cure Strategy for HIV (Abstract 9) **Denis-Claude Roy** *(CA)* – Phase II Study of Haploidentical Stem Cell Transplantation Using Ex Vivo Photodepletion of Donor Lymphocyte Infusions to Eliminate Anti-Host Reactivity Results in Low Relapse Rates and High Survival Rates: Final 2 Year Follow-Up (Abstract 10)	*Room 520CF*
	ADVANCED PRACTICE PROFESSIONALS (APP) TRACK SESSION 1 **Pharmacy Roles in Cellular Therapy & Hematopoietic Stem Cell Transplant** *1.25 AMA PRA Category 1 Credits™* ***Chair: Tobi Fisher** (US)* ***Speakers:*** **Rebecca Gonzalez** *(US)* – Old Drug-New Tricks: Post-transplant Cyclophosphamide in HSCT Setting: Key Opportunities for Clinical Pharmacists **Christina Bachmeier** *(US)* – Racing Towards the Finish Line with CAR-T Cell Therapy: The Pharmacist's Role in the Pit Crew	*Room 518A*
	QUALITY AND OPERATIONS TRACK SESSION 1 **Progress in the World of Standards for Cell and Gene Therapies** ***Chair: Sowmya Viswanathan** (CA)* ***Speakers:*** **Jiwen Zhang** *(US)* – Standards Coordinating Body (SCB): Advancing Standards for Regenerative Medicines **Claudia Zylberberg** *(US)* – Filling Information Gaps to Accelerate Commercialization: Two Case Studies in the Development of ISO Standards for Regenerative Medicine **Jonathan Campbell** *(UK)* – Standards Activities For Manufacturing Characterisation and Control of Cell-based Products **Yutaka Yanagita** *(JP)* – Standardization for Regenerative Medicine – Japan's Contribution	*Room 520BE*

Concurrent Sessions

Thursday, May 3

Concurrent Sessions	**STRATEGIES FOR COMMERCIALIZATION TRACK SESSION 1** **Future Proofing Your Supply Chain; Using the Lessons of the Past to Create Commercially Viable Logistics Platforms for the Future** ***Chair: Simon Ellison*** *(UK)* ***Speakers:*** **Sven Kili** *(UK)* – Lessons Learned from Currently Marketed Cell and Gene Therapies – Personal Reflections **Christina Yi** *(US)* – The Race Against Time – the Pioneering Supply Chain Behind the Delivery of PROVENGE, A Commercial Immunotherapy **Robert Preti** *(US)* – The Current and Future Landscape for Cell Therapy Deliverability: A CDMO Perspective	*Room 518BC*
	STRATEGIES FOR COMMERCIALIZATION TRACK SESSION 2 **How do Clinical Trial Design and Subsequent Learnings Impact Commercialization?** ***Chair: Uri Herzberg*** *(US)* ***Speakers:*** **Robert W. Mays** *(US)* – Applying Lessons from Early Stage Clinical Trials to the Pivotal Phase III MASTERS-2 Ischemic Stroke Study **Steven Fischkoff** *(US)* – Clinical Trial Data and Learnings Supporting Product Commercialization **Monica Luchi** *(US)* – Clinical Trial Design Incorporation of Commercial Considerations	*Room 520AD*
12:15–13:45	Lunch with Exhibits	*Exhibit Hall*
12:30–13:30	**CORPORATE TUTORIAL HOSTED BY BD BIOSCIENCES** **Improving Cell Therapy Production with Standardized and Consistent Flow Cytometry Assays and Sterile Fluorescence Activated Cell Sorting**	*Room 518BC*
12:30–12:45	**CORPORATE PRODUCT THEATRE HOSTED BY BECKMAN COULTER** **Get Lean: Streamlining Single Cell Analysis Workflow**	*Global Product Showcase (Exhibit Hall)*
12:45–13:00	**CORPORATE PRODUCT THEATRE HOSTED BY CCRM** **Developing Closed and Integrated Processes for Cell Therapy Manufacturing**	*Global Product Showcase (Exhibit Hall)*
13:00–13:15	**CORPORATE PRODUCT THEATRE HOSTED BY MILTENYI BIOTEC** **Automated Clinical-Scale of Gene-Engineered T cells Using the CliniMACS Prodigy**	*Global Product Showcase (Exhibit Hall)*
13:15–13:30	**CORPORATE PRODUCT THEATRE HOSTED BY BE THE MATCH** **Auditing, Onboarding, and Managing Apheresis and Marrow Collection Networks to Support Late-Stage Clinical Study and Commercial Launch of Cell and Gene Therapies**	*Global Product Showcase (Exhibit Hall)*
13:45–15:15	**PLENARY SESSION 2** **Mesenchyme Biology and Translational Use** *1.5 AMA PRA Category 1 Credits™* ***Chair: Jacques Galipeau*** *(US)* ***Speakers:*** **Martin Hoogduijn** *(NL)* – What Do We Really Know About MSC-Mediated Immune Regulation? **Bruno Péault** *(UK and US)* – Mesenchymal Stem Cells: Native Identity, Diversity, and Culture Induced Alterations **Duncan Stewart** *(CA)* – Gene-Enhanced Angiogenic Cell Therapy for Cardiovascular Diseases: An Update on the ENACT-AMI and SAPPHIRE Clinical Trials	*Plenary Hall*
15:15–15:45	Coffee Break with Exhibits	*Exhibit Hall*
15:15–15:45	**CORPORATE PRODUCT THEATRE HOSTED BY IRVINE SCIENTIFIC** **Chemically-Defined Culture Media for Advancing Cell-Based Immunotherapy Technology**	*Global Product Showcase (Exhibit Hall)*

Thursday, May 3

Concurrent Sessions

15:45–17:00

PLENARY BREAKOUT 1 – Endothelial Progenitor Cells
Room 519AB

1.25 AMA PRA Category 1 Credits™

***Chair: Duncan Stewart** (CA)*

Speaker:

Bernard Thébaud *(CA)* – EPC-based Therapies for Neonatal Pulmonary Hypertension

ORAL ABSTRACT PRESENTATIONS:

Amanda Olson *(US)* – A Phase I Trial of Mesenchymal Stem Cells Transfected with a Plasmid Secreting Interferon Beta in Advanced Ovarian Cancer (Abstract 11)
Christian Tebid *(CA)* – Cellular Therapy for Open Angle Glaucoma with Emphasis on Mesenchymal Stem Cells Secretome Induced Trabecular Meshwork Regeneration (Abstract 12)
Sowmya Viswanathan *(CA)* – Potential Anti-Inflammatory Mechanism of Action of Mesenchymal Stromal Cells in Osteoarthritis Patients Results in Overall Improvement in Pain and Symptoms (Abstract 13)

PLENARY BREAKOUT 2 – Translational Use of Mesenchymal Stem Cells
Room 520CF

1.25 AMA PRA Category 1 Credits™

***Chair: Bruno Péault** (UK and US)*

Jacques Galipeau *(US)* – Mesenchymal Stromal Cells – Addressing the Dissonance of Pre-Clinical Science and Clinical Development
Michael Matthay *(US)* – Mesenchymal Stromal Cells: Mechanisms and Safety for ARDS
Joanne Kurtzberg *(US)* – A Phase 3 Single-Arm, Prospective Study of Remestemcel-L, Ex-Vivo Cultured Adult Human Mesenchymal Stromal Cells, for the Treatment of Steroid Refractory Acute GVHD in Pediatric Patients

ORAL ABSTRACT PRESENTATION:

Renée Bazin *(CA)* – Warming Events and MSC Cryopreservation: Observations that Could Lead to the Development of Surrogate Tests for Immunosuppressive Activity (Abstract 15)

ISCT CHIEF SCIENTIFIC OFFICER ABSTRACT SHOWCASE
Room 518BC

***Chair: Daniel Weiss** (US)*

ORAL ABSTRACT PRESENTATIONS:

Shant Der Sarkissian *(CA)* – Identification of Differential Expression Phenotypes of CD133+ Stem Cells in Acute and Chronic Myocardial Infarct Patients and Specific Expression Pathways Underpinning Therapeutic Responsiveness in Regenerative Therapy (Abstract 16)
Gustavo Moviglia *(AR)* – Developing of Tissue Engineered Auricular Pavilion Able to Induce Host Perichondrium and Subdermal Tissue Integration. Preclinical Feasibility and Safety Study (Abstract 17)
Robert Nordon *(AU)* – Large-Scale, Single Cell RNA Sequencing Defines Novel Cellular Subsets Required for Cardiac Repair (Abstract 18)
Ioannis Papantoniou *(BE)*– Designing Microtissue Bioassemblies for Skeletal Regeneration: Healing Critical Size Long Bone Defects (Abstract 19)
Joel Simpson-Edin *(SE)* – Combining Electrospinning and 3D Printing for a New Generation of Nerve Guides (Abstract 20)

ADVANCED PRACTICE PROFESSIONALS (APP) TRACK SESSION 2
CAR T Immunotherapy: Science and Logistics
Room 518A

1.25 AMA PRA Category 1 Credits™

***Chair: Megan Cornelison** (US)*

Speakers:

Bambi Grilley *(US)* – Supporting Investigator Initiated Trials
Stanley Riddell *(US)* – Understanding and Managing Toxicities of CAR T cells

SCIENTIFIC AREA OF FOCUS – Stem Cell Biology and Expansion
Room 520AD

1.25 AMA PRA Category 1 Credits™

***Chair: John Rasko** (AU)*

Speakers:

Colleen Delaney *(US)* – Universal Donor Cord Blood Cell Therapies: Expanding Clinical Access and Improving Clinical Feasibility
Guy Sauvageau *(CA)* – Cellular Engineering with UM171: From Bedside to Benchtop
Shahin Rafii *(US)* – Vascular Niche Signals for Expansion of Engraftable Stem Cells

QUALITY AND OPERATIONS TRACK SESSION 2
Applying Change Within Controlled Systems and Processes
Room 520BE

***Chair: Rosemarie Bell** (AU)*

Speakers:

Steven Keizer *(CA)* – Defining the Change
Bangon Longsomboon *(US)* – Regulatory Expectations on Managing Change During R&D Through Clinical Product Manufacturing
Gerry McKiernan *(AU)* – Management of a Cell Expansion Platform Change During Clinical Trial Manufacturing

Thursday, May 3

17:00–18:30	**ADVANCED PRACTICE PROFESSIONALS TRACK SESSION 3** **What's New in Cord Blood Transplantation for Hematologic Malignancies** *1.5 AMA PRA Category 1 Credits™* ***Chair: Tobi Fisher** (US)* ***Speakers:*** **Juliet Barker** *(US)* – Intermediate Intensity Double Unit CBT as a Platform for Cellular Therapy **Valkal Bhatt** *(US)* – CBT: Day to Day Management of CBT Recipients: Graft Infusion and Specialized Pharmacology	*Room 518A*
17:15–19:15	**CORPORATE SYMPOSIUM HOSTED BY NOVARTIS ONCOLOGY** **What Makes CARs Work? – A Look Under the Hood**	*Plenary Hall*
18:00–19:30	POSTER SESSION 1	*Poster Hall*
19:00–21:00	**ISCT EARLY STAGE PROFESSIONALS (ESP) NETWORKING RECEPTION** *(by invitation only)*	*Westin Presidential Suite*
19:30–21:30	**ISCT INDUSTRY NETWORKING EVENT** *(by invitation only)*	*Offsite*

Friday, May 4

06:30–07:30	**ISCT 2018 5K RUN THE RIVER EVENT** *(Registration Required)*	*Offsite*
07:00–18:00	Registration	*Level 5 Foyer*
07:00–17:00	Speaker Services	*Room 521C*
Concurrent Sessions 07:30–08:30	**HOT TOPIC SESSION 3 – Immunologic Sculpting of T Cell and Stem Cell Grafts** *1.0 AMA PRA Category 1 Credits™* ***Chair: Denis-Claude Roy** (CA)* ***Speakers:*** **Alice Bertaina** *(US)* – Haploidentical Transplant in the Era of Graft Engineering: What's New in Pediatrics **Denis-Claude Roy** *(CA)* – Anti-infection and Anti-leukemia Donor T cells Without GVHD: "Mission Possible" **Isabelle Rivière** *(US)* – Advances in CAR T-Cell Therapy: The CD19 CAR Paradigm	*Room 520BE*
	HOT TOPIC SESSION 4 – Oncolytic Viruses as Cancer Therapeutics *1.0 AMA PRA Category 1 Credits™* ***Chair: John Bell** (CA)* ***Speakers:*** **John Bell** *(CA)* – Development of a Novel Rhabdovirus Platform for the Treatment of Cancer **Khalid Shah** *(US)* – Oncolytic Virus Engineered Cell Based Therapeutics for Cancer	*Room 520CF*
	ORAL ABSTRACT PRESENTATION: **Yashbir Singh** *(TW)* – Functional Annotation and Identification of Putative Drug Target in Strain Ankara (Abstract 21)	
	CORPORATE TUTORIAL HOSTED BY AMERICAN SOCIETY OF MECHANICAL ENGINEERS (ASME) **Training the Engineering Workforce for Cellular Therapy Manufacturing**	*Room 518BC*
08:45–10:15	**PLENARY SESSION 3 – Exosomes** *1.5 AMA PRA Category 1 Credits™* ***Chair: Daniel J. Weiss** (US)* ***Speakers:*** **Eva Rohde** *(AT)* – Manufacturing and Characterization of MSC-EVs for Clinical Testing **Linda Marban** *(US)* – Exosomes – CAP-2003 **Sai-Kiang Lim** *(SG)* – MSC Exosomes and Small EVs: A Comparative Review	*Plenary Hall*
10:15–10:45	Coffee Break with Exhibits	*Exhibit Hall*
	CORPORATE PRODUCT THEATRE HOSTED BY BIOLAMINA **CTG BioLaminin™ 521 – a Biologically Relevant Culture Matrix, Enabling Pre-clinical Research Protocols to be Translated and Used for Clinical Trials**	*Global Product Showcase (Exhibit Hall)*

Friday, May 4

Concurrent Sessions

10:45–12:15

PLENARY BREAKOUT 1 – Exosome Technology

Room 518A

1.5 AMA PRA Category 1 Credits™

Chair: Eva Rohde

Speakers:

Chulhee Choi *(KR)* – Exosome Engineering for Delivery of Therapeutic Proteins: Principles and Applications
Randolph Corteling *(UK)* – Production of Functionally Bioactive EVs from an Immortalized Human Neural Stem Cell Line and their Therapeutic Application

ORAL ABSTRACT PRESENTATIONS:

Anastasia Cheng *(CA)* – Licensing Increases The Quantity and Immunomodulatory Cargo of Mesenchymal Stromal Cell Exosomes (Abstract 22)
Jeremiah Oyer *(US)* – NK Cell Expansion With Ex21-Exosomes (Abstract 23)

PLENARY BREAKOUT 2
Lessons Learned During CAR T Commercial Roll Out at Clinical Sites

Room 520CF

Chair: Sarah Nikiforow *(US)*

Speakers:

Solveig Ericson *(US)* – Preparing Sites to Enter the Commercial CAR T Setting: Focus on Clinical Readiness
Diane Parks *(US)* – Best Practices – Supporting Logistical Accounts Transitions for Commercial CAR T Distribution
Sarah Nikiforow *(US)* – Growing Pains in an Academic CAR T Program, Including Transition from Clinical Trials to Commercial Products

ISCT PRESIDENTIAL TASK FORCE SESSION
Tailoring Communication on Cell Therapies Between Scientific Societies, Regulations, Bioethical Needs and Patient Perspectives

Room 519AB

Chair: Massimo Dominici

Speakers:

Massimo Dominici
Peter Marks *(US)* – FDA Perspectives on Stem Cell Therapies
Nadine Kolas *(CA)* – Regulation of Autologous Cell Therapies in Canada
Harold Atkins *(CA)* – A Physician's Perspective on Unproven Cell Therapies: How Hope Has Become a Commodity
Aaron Levine *(US)* – Ethical Considerations in Communication Surrounding Cellular Therapies

SCIENTIFIC AREA OF FOCUS – NK Cell Therapy: A New Frontier Revisited

Room 520AD

1.5 AMA PRA Category 1 Credits™

Chair: Armand Keating *(CA)*

Speakers:

Sarah Cooley *(US)* – Novel Strategies to Activate NK Cells to Treat Cancer
Katy Rezvani *(US)* – Armored CAR NK Cells for the Treatment of Lymphoid Malignancies
Armand Keating *(CA)* – Cell Therapy with Permanent NK Cell Lines

QUALITY AND OPERATIONS TRACK SESSION 3
Regulation of MTMMS and ATMPS: Manufacturing, Non-Clinical, and Clinical Perspectives

Room 520BE

Chair: Shirley Bartido *(US)*

Speakers:

Shirley Bartido *(CA)* – GMP Manufacturing Considerations for Advanced Therapeutic Cellular Products
Julianne Smith *(FR)* – Non-clinical Considerations in the Development of Cell and Gene Therapies
Elena Spanjaard *(US)* – Regulatory Challenges and Opportunities for Clinical Development of Cell and Gene Therapies

STRATEGIES FOR COMMERCIALIZATION TRACK SESSION 3
Tools and Automation Solutions for Accelerating Cell and Gene Therapy Development

Room 518BC

Chair: Julie Murrell *(US)*

Speakers:

Qasim Rafiq *(UK)*
Jon Rowley *(US)* – Technologies for Radically Reducing Development Timelines of hMSC-based Therapeutic Products
Gregory Russotti *(US)* – Strategies and Methodologies for Selecting and Implementing New Technologies for Cell Therapy Processes

Friday, May 4

12:15–13:45	Lunch with Exhibits	*Exhibit Hall*
12:30–13:30	**CORPORATE TUTORIAL HOSTED BY MACOPHARMA** **The First Clinical Grade Virally Inactivated Human Platelet Lysate, MultiPL'i: a Powerful, Safe and Standardized Alternative to FBS to Expand Cell Therapy Products**	*Room 518BC*
12:30–13:00	**CORPORATE PRODUCT THEATRE HOSTED BY COOK REGENTEC** **Different Makes The Difference: The CellSeal® System**	*Global Product Showcase (Exhibit Hall)*
13:00–13:15	**CORPORATE PRODUCT THEATRE HOSTED BY CELLCAN REGENERATIVE MEDICINE AND CELL THERAPY NETWORK** **More than 4800 days of a Wonderful Second Life! William Brock's Inspiring Story!**	*Global Product Showcase (Exhibit Hall)*
13:15–13:30	**CORPORATE PRODUCT THEATRE HOSTED BY CELLCAN REGENERATIVE MEDICINE AND CELL THERAPY NETWORK** **How Well Do You Know Canada's Cell & Gene Manufacturing Landscape?**	*Global Product Showcase (Exhibit Hall)*
13:45–15:15	**PLENARY SESSION 4** **Reimagining Cancer Care and Delivering on the Promise of CAR-T Therapies** ***Chair: Miguel Forte*** *(BE)* ***Speakers:*** **Carl June** *(US)* – CAR T: Current Status and Perspectives from the Academic Lens **Pascal Touchon** *(US)* – Reimagining Cancer Care and Delivering Kymriah to Patients **Diane Parks** *(US)* – Making History - Commercial Launch of CAR-T Therapy	*Plenary Hall*
15:15–15:30	Coffee Break with Exhibits	*Exhibit Hall*
	CORPORATE PRODUCT THEATRE HOSTED BY AVENTACELL BIOMEDICAL CORP. **What's New with Human Platelet Lysate?**	*Global Product Showcase (Exhibit Hall)*
Concurrent Sessions 15:30–17:00	**PLENARY BREAKOUT 1 – Pre-Clinical Application of Exosomes** *1.5 AMA PRA Category 1 Credits™* ***Chair: Linda Marban*** *(US)* ***Speakers:*** **Lambros Kordelas** *(DE)* – Heterogeneity of MSCs and Clinical Potential of their Extracellular Vesicles **Marie-Josée Hébert** *(CA)* – Apoptotic Exosomes: New Players in the Regulation of the Immune Response **ORAL ABSTRACT PRESENTATIONS:** **Mirabelle Ho** *(CA)* – The X-Factor Involved in Apoptosis and Proliferation - Reciprocal Exosome Cross-Talk Between Lymphangioleiomyomatosis-Derived Smooth Muscle Cells (LAM-SMCs) and Endothelial Cells (ECs) Mediates EC Network Disruption/Apoptosis and Promotes LAM-SMC Growth (Abstract 24) **Wei Seong Toh** *(SG)* – MSC Exosomes Alleviate Pain and Degeneration in a Rat Model of Temporomandibular Joint Osteoarthritis (Abstract 25)	*Room 519AB*
	PLENARY BREAKOUT 2 **Market Access & Reimbursement of CAR T Therapies** ***Chair: Dawn Driscoll*** *(AU)* ***Speakers:*** **Rocio Manghani** *(US)* – Market Access Learning from the First CAR-T in the Adult Population **Douglas Olson** *(US)* – CAR-T Therapy, A Patient's Perspective **Daniel Ollendorf** *(US)* – Valuing Cellular, Regenerative, and Gene Therapies: The Intersection of Promise, Uncertainty, and Cost **Tammy Clifford** *(CA)* – Implementing Gene Therapies in the Canadian Health System: What Does the Future Look Like?	*Room 520CF*

Friday, May 4

Concurrent Sessions

15:30–17:00

ISCT 2018 ORGANIZING COMMITTEE ABSTRACT SHOWCASE

Room 520AD

***Co-Chairs: Sarah Nikiforow** (US) **and Rachele Ciccocioppo** (IT)*

ORAL ABSTRACT PRESENTATIONS:

Kai Nesemann *(DE)* – First Rapid Final Release Test of ATMPs Prior To Treatment – Validation of a Robust and Highly Sensitive qPCR Test For Total Bacteria (Abstract 26)
Jennifer Moseley *(US)* – Cardiosphere-Derived Cells Show Strong Immunomodulatory Activity and Improve Muscle Physiology When Systemically Delivered in a DMD Mouse Model (abstract 27)
Sarah Nikiforow *(US)* – 5-Year Performance Data and Robust Flexibility of a Computerized Physician Ordering System For Collection, Processing, and Administration of Cellular Therapy Products (Abstract 28)
Ahmad Galuta *(CA)* – Direct Comparison of Adult Human and Rat Spinal Cord Stem Cell Behavior (Abstract 368)
Dani Offen *(IL)* – Towards Gene-Editing Treatment For Alzheimer's Disease: ApoE4 Allele-Specific Knockout Using a CRISPR Cas9 Variant (Abstract 30)
Ngaire Elwood *(AU)* – Expanding Cellular Therapies Through Provision of a Cord Blood – Derived iPSC Haplobank (Abstract 370)

EARLY STAGE PROFESSIONALS SESSION 1

Room 518A

Translating Cell and Gene Therapy Products from Bench to Bedside

***Co-Chairs: Patrick Hanley** (US) **and Fernanda Masri** (UK)*

Speakers:
Kenneth Micklethwaite *(AU)* – Cell Therapy Translation- Pathways and Pitfalls
Paul Eldridge *(US)*
Bambi Grilley *(US)* – Developing and Implementing Investigator Initiated Trials: Practical Considerations
Sadik Kassim *(US)* – The Path From Lab Bench Discovery to Startup Company

QUALITY AND OPERATIONS TRACK SESSION 4

Room 520BE

Day to Day Operations of a Stem Cell Lab

***Chair: Heather Garrity** (US)*

Speakers:
Karl Stasko *(US)* – Making Novel Normal: Incorporating Commercial CAR T into the Daily Operations of a Lab
Joseph Schwartz *(US)*
Christine Rosati *(US)* – Implementing CAR-T Program

STRATEGIES FOR COMMERCIALIZATION TRACK SESSION 4

Room 518BC

21st Century Global Regulatory Impact with Divergent Markets on Commercialization

***Chair: Julie Allickson** (US)*

Speakers:
Flagg Flanagan *(US)* – DiscGenics Overview. Regulatory Pathways in the US and Japan
John Martin *(AU)* – 21st Century Global Regulatory Impact with Divergent Markets on Commercialization
Rainer Marksteiner *(AT)* – European ATMP regulation: Milestone or Hindrance?
Colin Novick *(JP)*

Panelists:
Julie Allickson *(US)*, **Flagg Flanagan** *(US)*, **John Martin** *(AU)*, **Rainer Marksteiner** *(AT)*, **Colin Novick** *(JP)*, **Michael Mendicino** *(US)*

17:00–18:30 — POSTER SESSION 2 — *Poster Hall*

17:15–17:45 — **CORPORATE MASTER CLASS HOSTED BY THERMO FISHER SCIENTIFIC**
Identifying a Lentiviral Production Platform to Achieve Cost-Effective Scalable Manufacturing — *Global Product Showcase (Exhibit Hall)*

19:00–23:00 — **ISCT 2018 GALA** *Tickets Required* — *Offsite*
Buses leave from Westin Hotel starting at at 18:30

Saturday, May 5

	07:30–15:00	Registration	*Level 5 Foyer*
	07:30–15:00	Speaker Services	*Room 521C*
Concurrent Sessions	08:00–09:00	**HOT TOPIC SESSION 5** *Room 519AB* **Solid Organ Transplantation: Tolerance Induction and Tregs Immunotherapy** *1.0 AMA PRA Category 1 Credits™* ***Chair: Stephan Busque** (US)* ***Speakers:*** **Stephan Busque** *(US)* – Tolerance Induction After Kidney transplantation: Overview and Stanford's Experience **Joseph Leventhal** *(US)* – Therapeutic Cell Transfer to Achieve Transplantation Tolerance: from Facilitating Cells to Regulatory T cells	
		HOT TOPIC SESSION 6 *Room 520CF* **Biomaterials and Clinical Uses** *1.0 AMA PRA Category 1 Credits™* ***Chair: May Griffith** (CA)* ***Speakers:*** **May Griffith** *(CA)* – Biomimetic Materials and Cornea Regeneration: from Bench to Bedside and Back **Lucie Germain** *(CA)* – Tissue Engineered Corneal Epithelium for Clinical Application **Jöns Hilborn** *(SE)* – Biomaterials for Engineering of Bone **Eve Tsai** *(CA)* – Biomaterials to Translate Promising Therapies for Neural Regeneration to Humans	
		QUALITY AND OPERATIONS TRACK SESSION 5 *Room 520BE* **Data Integrity - Requirements, Opportunities, Management, Reconciliation** ***Chair: Gabrielle O'Sullivan** (AU)* ***Speakers:*** **James Blackwell** *(US)* – The Future of Data Integrity for Cellular Therapies – Why You Need to Think About It Now **Robert Margolin** *(US)* – Using Software to Manage Multicentre Advanced Therapy Supply Cycles **Zlatibor Velickovic** *(AU)* – Data Integrity Challenges and Solutions in a Public Hospital Based Cell Therapy Manufacturing Facility **Alex Denoon** *(UK)* – Reconciling Data Integrity Requirements and the GDPR	
		STRATEGIES FOR COMMERCIALIZATION TRACK SESSION 5 *Room 518BC* **Managing Patient Expectations and Reimbursement/Patient Advocacy/Market Access** ***Chair: Gerhard Bauer** (US)* ***Speakers:*** **Gerhard Bauer** *(US)* – Making Cellular Therapy Products Available in the North American Market **Dawn Driscoll** *(AU)* – Patient Access to CAR-T Before Approval **Douglas Olson** *(US)* – CAR-T Therapy, A Patient's Perspective	
	09:15–10:45	**PLENARY SESSION 5** *Plenary Hall* **ISCT-ASGCT Joint Session: Genome Editing for Benign and Malignant Diseases** *1.5 AMA PRA Category 1 Credits™* ***Co-Chairs: Donald Kohn** (US) **and Helen Heslop** (US)* ***Speakers:*** **Matthew Porteus** *(US)* – Gene Editing of Hematopoietic Stem and Progenitor Cells **John Zaia** *(US)* – Genome Editing with ZFN using Blood Progenitor Cells in HIV/AIDS **Michael Holmes** *(US)* – Highly Efficient and Specific Multiplexed Gene Editing in T Cells Using Enhanced Zinc-finger Nucleases (ZFNs) Enables Strategic Engineering of Allogeneic T Cell Immunotherapies	
	10:45–11:00	Coffee Break	*Level 5 Foyer*

Saturday, May 5

Concurrent Sessions

11:00–12:00

PLENARY BREAKOUT 1 – Editing to Enhance Cell Therapy – Universal T Cells

Room 519AB

1.0 AMA PRA Category 1 Credits™

***Chair: Helen Heslop** (US)*

Speaker:

Andrew Scharenberg *(US)* – Engineering Regulatory T Cells for Inflammatory Disorders

ORAL ABSTRACT PRESENTATIONS:

Vincenzo Di Cerbo *(UK)* – Single Cell Analysis of Lentiviral Integration To Support Ex-Vivo Gene Modified Cell Therapy Development (Abstract 29)
Yelena Boccacci *(CA)* – CRISPR-Driven Modeling of Clinically Relevant Genetic Variants in Hematopoietic Stem and Progenitor-Derived Erythroid Cells (Abstract 369)

PLENARY BREAKOUT 2 – Ex Vivo Editing – HSC and iPSC

Room 520CF

1.0 AMA PRA Category 1 Credits™

***Chair: Matthew Porteus** (US)*

Speaker:

Donald Kohn *(US)* – Improving Homology-Directed Repair in Hematopoietic Stem Cells

ORAL ABSTRACT PRESENTATIONS:

Houria Bachtarzi *(UK)* – How Genetically Modified Organism (GMO) Regulations Impact On The Development of Genetically Modified Cell-Based Therapies in Europe, The U.S. and Japan (Abstract 31)
Roberta Mazzieri *(AU)* – Adoptive Transfer of Genetically Engineered Monocytes for the Tumour Targeted Delivery of IFN-Alpha (Abstract 32)

QUALITY AND OPERATIONS TRACK SESSION 6
Challenges of cGMP Clean Room Facility for Cell Therapy Manufacturing in an Academic Setting

Room 520BE

***Chair: Nadim Mahmud** (US)*

Speakers:

Nadim Mahmud *(US)* – Challenges to Build and Validate a cGMP Facility Without Interruption of Ongoing Graft Processing Services
Chy-Anh Tran *(US)* – Risk Management for cGMP Academic Facility
Aisha Khan *(US)* – How to Set up a GMP Compliant Clean Room for an Academic Vector Production

STRATEGIES FOR COMMERCIALIZATION TRACK SESSION 6
Growing the Pie: Has Investor Interest Increased for CGT post-approval of Kymriah, Yescarta and Luxturna?

Room 518BC

Chair: William Milligan

Speakers:

Patrick Rivers *(US)* – A Whale Swims into a Pond... Cell and Gene Therapy Hit the Market - Now What?
Didier Leconte *(CA)* – Venture Capital Views on Investments in CGTs
Matthew Durdy *(UK)* – The European Perspective

11:00–12:30

EARLY STAGE PROFESSIONALS SESSION 2
Young Investigator Abstract Showcase

Room 520AD

***Co-Chairs: Alireza Abazari** (US) **and George Hucks** (US)*

ORAL ABSTRACT PRESENTATIONS:

Claudia Raggi *(CA)* – Safety of Stem Cell-Derived Encapsulated Liver Tissue To Treat Liver Failure: Immune-Isolation and Absence of Foreign Body Reaction Or Tumor Formation Upon Transplantation Without Immunosuppression (Abstract 33)
Sabiha Hacibekiroglu *(CA)* – Combined Cell and Gene Therapy Towards The Treatment of Age-Related Macular Degeneration and Diabetic Retinopathy (Abstract 34)
Alexander Johnston *(CA)* – First Trimester Human Umbilical Cord Perivascular Cells (FTM HUCPVCs) Expanded With cGMP Compliant Human Platelet Outperform Conventional and Older MSC Sources As Regenerative Therapy in a Rat Myocardial Infarction (MI) Model (Abstract 35)
Ewelina Kurtys *(CA)* – In Vivo Tracking of CAR-T By [^{18}F]BF4- PET/CT in Human Breast Cancer Xenografts Reveals Differences in CAR-T Tumour Retention (Abstract 36)

Speaker:

John Barrett *(US)* – How to Be Successful at Publishing your Work

Saturday, May 5

12:00–13:30	Lunch	*Level 5 Foyer*
12:15–13:15	ISCT Annual General Business Meeting	*Plenary Hall*
13:30–15:00	**PLENARY SESSION 6 – iPSC Regenerative Medicine** *1.5 AMA PRA Category 1 Credits™* ***Chair: Andras Nagy** (CA)* ***Speakers:*** **Maria Mirotsou** *(US)* – Translating Stem Cell Biology into Regenerative Medicine **Mahendra Rao** *(US)* – Making iPSC Cell Derived Products **Andras Nagy** *(CA)* – A Solution for Cell Therapy Safety and Engineered Allotolerance	*Plenary Hall*
15:00–15:15	Coffee Break	*Platinum Suite*
Concurrent Sessions 15:15–16:30	**PLENARY BREAKOUT 1 – iPSC and Organogenesis** *1.25 AMA PRA Category 1 Credits™* ***Chair: Maria Mirotsou** (US)* ***Speakers:*** **Timothy Kieffer** *(CA)* – Making Functional Islet Cells from Human Pluripotent Stem Cells **Gilbert Bernier** *(CA)* – Modeling Human Retinal Degenerative Diseases Using iPSC-derived 3D Photoreceptor Sheets **ORAL ABSTRACT PRESENTATIONS:** **Jaap Jan Boelens** *(NL)* – Rapid and Robust Cd4+ and Cd8+ T-, Nk-, B- and Monocyte Cell Reconstitution After Nicotinamide Expanded Cord Blood (nicord) Transplantation (Abstract 37)	*Room 519AB*
	PLENARY BREAKOUT 2 – iPS Generation and Skin and Lung Applications *1.25 AMA PRA Category 1 Credits™* ***Chair: Mahendra Rao** (US)* ***Speaker:*** **Jean Tang** *(US)* – Collagen VII Skin Grafts for Chronic RDEB Wounds **ORAL ABSTRACT PRESENTATIONS:** **Holly Anderson** *(US)* – Safety Considerations in the Generation of Clinical Grade Autologous iPS Cell Lines (Abstract 38) **Miriel Ho** *(CA)* – Close Neighbors in the Niche: Paracrine Signals from Endothelial Cells Promote Alveolar Epithelial Differentiation of Induced Pluripotent Stem Cell-derived Lung Progenitors (Abstract 39) **Rebecca Lim** *(AU)* – Allogeneic Amniotic Epithelial Cells for Established Bronchopulmonary Dysplasia in Premature, Low Birthweight Infants: A first-in-human Safety Trial (Abstract 40)	*Room 520CF*
	QUALITY AND OPERATIONS TRACK SESSION 7 **Regulatory Expectations for the Manufacturing Control Strategy** ***Co-Chairs: Rosemarie Bell** (AU) **and Sowmya Viswanathan** (CA)* ***Speakers:*** **Christopher Storbeck** *(CA)* – CMC Challenges in Cell and Gene Therapies: A Health Canada Perspective **Karin Hoogendoorn** *(NL)* – Introduction to Process Control Strategy **Paula Salmikangas** *(UK)* – Design and Optimisation of Process & Product Controls for Cell-based Products	*Room 520BE*
	STRATEGIES FOR COMMERCIALIZATION TRACK SESSION 7 **ISCT-BPSA Joint Session: Next Generation Manufacturing for Cell and Gene Therapies** ***Co-Chairs: Ohad Karnieli** (IL) **and Eric Isberg** (US)* ***Speakers:*** **Dominic Clarke** *(US)* – Defining Single-Use Systems for Cell & Gene Therapy Manufacturing **Gary Pigeau** *(CA)* – Bioreactor Process Correlations and Sample-free Cell Expansion **Randy Schweickart** *(US)* – Supervisory Control Automation for Next Generation Cell Therapy Manufacturing **Panelists: Ohad Karnieli** *(IL)*, **Eric Isberg** *(US)*, **Dominic Clarke** *(US)*, **Gary Pigeau** *(CA)*, **Randy Schweickart** *(US)*, **Shannon Eaker** *(US)*	*Room 518BC*

Plenary and Plenary Breakout Session Summaries

Presidential Plenary Session – Cancer Immunotherapy

Thursday, May 3, 9:00-10:30 ***Plenary Hall***

Chair: Catherine Bollard

With Cellular ImmunoTherapy in the spotlight with recent FDA approvals, the Presidential Plenary will focus on Immunotherapy where we are fortunate to have attracted leaders in the field to speak including Stanley Riddell, Crystall Mackall and one of ISCT's Past Presidents, Robert Negrin. These speakers will focus on the development of novel cell therapeutics for cancer. Dr Riddell will speak on "Beyond CD19-CARs for Hematologic Malignancies", Dr Mackall will speak on "Cell Therapies for Solid Tumors" and Dr Negrin will speak on "Targeting the Microenvironment".

Strategies for Improving Efficacy and Safety of CAR T Cells for Cancer Therapy

Stanley Riddell

Advances in synthetic biology and adoptive T cell transfer are making inroads in cancer therapy. Genetically modified T cells that express synthetic chimeric antigen receptors (CARs) have had success in patients with advanced B cell malignancies. Our lab conducted the first clinical trials in which the CD4 and CD8 T cell composition of the CD19 CAR-T cell product is uniform in all patients. This approach identified CAR-T cell dose/response and dose/toxicity relationships not apparent in prior studies, and improved the therapeutic index in B cell tumors. The outgrowth of tumor cells with low or absent antigen expression remains an obstacle for durable remissions in some patients. a larger challenge is to extend CAR-T cell therapy to common solid tumors, where identifying molecules expressed on cancer cells that can be targeted safely remains an obstacle. We developed CARs that target the receptor tyrosine kinase like orphan receptor ROR1 expressed in many cancers including non small cell lung cancer (NSCLC) and triple negative breast cancer (TNBC). ROR1 is expressed on some normal tissues and we have used a mouse model to examine how various logic gated CARs might redirect selective recognition of tumor cells and not normal tissues.

CAR T cells for Cancer: Progress and Challenges

Crystal Mackall

The impressive response rates of chimeric antigen receptor (CAR) modified T cells in B cell malignancies illustrate their remarkable capacity to kill chemoresistant cancer. However, much work remains to be done if CAR T cells are to cure a high fraction of patients treated and if this potency is to be extended to other chemoresistant cancers. Experience in B cell malignancies has identified primary mechanisms of resistance to CAR T cells as antigen escape and T cell failure. This presentation will discuss factors contributing to antigen neg/lo escape and approaches to overcome this challenge. I will also discuss work underway to render CAR expressing less susceptible to T cell exhaustion, which appears to be the major cause of T cell failure in this setting.

Allogeneic Cells with Limited GVHD Potential for Cancer Immunotherapy

Robert Negrin

In this presentation I will discuss the role of different effector cell populations that have limited capacity for induction of graft vs host disease and can therefore be used in the allogeneic setting. These cell populations will include natural killer, invariant natural killer T cells and cytokine induced killer cells. The biology of these different cell populations will be illustrated with clinical studies.

Plenary Breakout 1 – Cancer Immunotherapy I

Thursday, May 3, 11:00-12:15 ***Room 519AB***

Chair: Crystal Mackall

ORAL ABSTRACT PRESENTATIONS:

Novel CAR T that Targets MUC1* Not Full-Length MUC1 for Treatment of Solid Tumor Cancers (Abstract 1)
Cynthia Bamdad

Engineering the Tgfβ Receptor to Enhance the Therapeutic Potential of Natural Killer Cells as An Immunotherapy For Neuroblastoma (Abstract 2)
Rachel Burga

Factors Promoting CD19-Negative Relapses Following CAR19T Cell Therapy (Abstract 3)
Alla Dolnikov

T Cells Engineered With T-Cell Antigen Coupler (TAC) Receptors Display Robust Efficacy Against Solid and Liquid Tumor Xenografts in The Absence of Any Toxicity (Abstract 4)
Christopher Helsen

Simultaneous Dual CAR Expression to Prevent Relapse in Pre-B Acute Lymphoblastic Leukemia (Abstract 5)
William Lemieux

Plenary Breakout 2 – Cancer Immunotherapy II

Thursday, May 3, 11:00-12:15 ***Room 520CF***

Chair: Robert Negrin

ORAL ABSTRACT PRESENTATIONS:

BCMA-Specific TAC Receptor-Engineered T Cells For Multiple Myeloma (Abstract 6)
Ksenia Bezverbnaya

shRNA-Mediated TCR Knockdown as a Foundation For Allogeneic CAR19 T-Cells Generated By Single-Step Genetic Modification with the piggyBac Transposase (Abstract 7)
David Bishop

Modulation of Co-Signaling For Improved Ex Vivo Human Antigen-Specific T Cells Generation For Immunotherapy (Abstract 8)
Shirin Lak

HIV-Specific T Cells Can Be Generated Against Conserved Non-Escaped HIV Epitopes For Use in a Phase I Clinical Trial: Pre-Clinical Validations and Implications for a Cure Strategy For HIV (Abstract 9)
Shabnum Patel

Phase II Study of Haploidentical Stem Cell Transplantation Using Ex Vivo Photodepletion of Donor Lymphocyte Infusions to Eliminate Anti-Host Reactivity Results in Low Relapse Rates and High Survival Rates: Final 2 Year Follow-Up (Abstract 10)
Denis-Claude Roy

Plenary Session 2 – Mesenchyme Biology and Translational Use

Thursday, May 3, 13:45-15:15 ***Plenary Hall***

Chair: Jacques Galipeau

Mesenchymal stromal cells and related culture adapted cell pharmaceuticals have met important regulatory milestones in the past year with both EMA and likely FDA approvals for clinical use in the making. as the filed strives to develop MSC v2.0, understanding of ontogeny, biology and mechanism of action will inform on how best to improve potency and use of these cell products in expanded clinical applications. The remarkable observation that killed MSCs effect a response from treated mice suggests important unrecognized cell-function autonomous effects of MSC therapy. a point to be addressed by Dr Hoogduijn. On a more basic biology perspective, the deciphering of pericyte form and function in vivo provides important insights on recruitment of endogenous progenitors to affect an injury response. Dr Péault will highlight the biology of vascular fraction-derived MSC-like pericytes and how this knowledge can inform development of this tissue source for translational use. in closing, the translational use of gene engineered endothelial cells foreshadows the development of MSC engineered counterparts, and the clinical lessons learnt and shared by Dr Stewart will provide guidance to the field as well.

What Do We Really Know About MSC-Mediated Immune Regulation?

Martin Hoogduijn

In my presentation I will discuss the known immunoregulatory properties of MSC and link them to observations made after infusing MSC for therapy concerning survival and biological activity of the cells. It turns out there are some considerable discrepancies. I will discuss the effects of infusion of inactivated MSC and compare them to the effects of fully active MSC. I will end by proposing a model system to be used for finding out the real immunomodulatory mechanisms of action of MSC.

Mesenchymal Stem Cells: Native Identity, Diversity, and Culture Induced Alterations

Bruno Péault

Mesenchymal stem cells were indirectly selected by long-term culture for decades before their innate perivascular identity was revealed. Stringently purified peri-endothelial pericytes and stromal cells located in the tunica adventitia of arteries and veins become spontaneously re-programmed into MSCs in vitro. Single cell transcriptome analysis has shown that perivascular presumptive MSCs are ordered as a hierarchy dominated by developmentally more primitive adventitial cells. a proteomic approach has also revealed the expression by perivascular cells of novel markers which typify subsets of functionally distinct presumptive MSCs, in terms of proliferation potential and progenitor ability. in order to understand the molecular transition between native perivascular cells and their MSC descent in culture, total RNA was sequenced from both sorted human perivascular cells and the MSC progeny thereof after 4 passages in vitro. Systematic comparison showed that culture results in dramatic changes in gene expression, up to 33% of all genes expressed becoming modified. This was confirmed by comparing the growth factors and cytokines secreted by either intact perivascular cells or their MSC progeny, which also considerably differ. The general conclusion of these studies is that MSC phenotype and function are largely imposed by in vitro culture.

Gene-Enhanced Angiogenic Cell Therapy for Cardiovascular Diseases: an Update on the ENACT-AMI and SAPPHIRE Clinical Trials

Duncan Stewart

Adult progenitor and stem cells have been used to promote cardiac and blood vessel regeneration but randomized clinical trials have yielded variable results for clinical efficacy. Barriers to the efficacy of cell therapies include limited cell retention or engraftment, heterogeneity and marginal potency of many cell products, and the deleterious effect of host factors of the activity of autologous cell therapies. Our group has optimized the selection and manufacture of a well characterized mononuclear cell product called early outgrowth endothelial progenitor cells (EPC), derived from non-mobilized apheresis collections. to enhance proangiogenic potency, autologous EPCs (aka circulating angiogenic cells) are transiently transfected to overexpress endothelial NO-synthase (eNOS). The ENhanced Angiogenic Cell Therapy for Acute Myocardial Infarction (ENACT-AMI) trial is a multicenter double-blind study with 3-way randomization to saline (control), transfected and non-transfected EPCs delivered up to 30 days post AMI. Change in global EF by MRI is the primary endpoint, and the trial is about half-way to the enrolment target of 100 patients. The Study of Angiogenic cell therapy for Progressive Pulmonary Hypertension: Intervention with Repeat dosing of eNOS-enhanced EPCs (SAPPHIRE) is Canada-wide muticentre trial which has recently initiated enrolment. Up to 8 doses of the eNOS-enhanced EPCs (160M cells in total) will be delivered IV over one year in an innovative 3-arm cross over.

Plenary Breakout 1 – Endothelial Progenitor Cells

Thursday, May 3, 15:45-17:00 ***Room 519AB***

Chair: Duncan Stewart

EPC-based Therapies for Neonatal Pulmonary Hypertension

Bernard Thébaud

- Describe the importance of angiogenesis in lung development.
- Learn about lung resident endothelial colony-forming cells (ECFCs).
- Present therapeutic potential of ECFCs in lung disease.

ORAL ABSTRACT PRESENTATIONS:

A Phase I Trial of Mesenchymal Stem Cells Transfected With a Plasmid Secreting Interferon Beta in Advanced Ovarian Cancer (Abstract 11)
Amanda Olson

Cellular Therapy for Open Angle Glaucoma With Emphasis On Mesenchymal Stem Cells Secretome Induced Trabecular Meshwork Regeneration (Abstract 12)
Christian Tebid

Potential Anti-Inflammatory Mechanism of Action of Mesenchymal Stromal Cells in Osteoarthritis Patients Results in Overall Improvement in Pain and Symptoms (Abstract 13)
Sowmya Viswanathan

Plenary Breakout 2 – Translational Use of Mesenchymal Stem Cells

Thursday, May 3, 15:45-17:00 ***Room 520CF***

Chair: Bruno Péault

Mesenchymal Stromal Cells – Addressing the Dissonance of Pre-Clinical Science and Clinical Development

Jacques Galipeau

- Analyze and understand comparative MSC cell biology in mouse and humans
- Analyze and understand failure and successes of MSC in human clinical trials
- Understand opportunities in improving MSC outcomes in human clinical trials

Mesenchymal Stromal Cells: Mechanisms and Safety for ARDS

Michael Matthay

- The preclinical evidence showing efficacy of mesenchymal stromal cells (MSCs) for reducing acute lung injury in experimental models of ARDS.
- The translational regulatory and practical steps to obtaining FDA and NIH support for early clinical trials.
- The results of two safety trials - phase 1 dose escalation trial in 9 ARDS patients and a phase 2a randomized double blind trial for safety in moderate to severe ARDS.

A Phase 3 Single-Arm, Prospective Study of Remestemcel-L, Ex-Vivo Cultured Adult Human Mesenchymal Stromal Cells, for the Treatment of Steroid Refractory Acute Gvhd in Pediatric Patients

Joanne Kurtzberg

- Review the clinical experience using MSC in children with GVHD
- Review the design of study MSC-100-IV

ORAL ABSTRACT PRESENTATION:

Warming Events and MSC Cryopreservation: Observations That Could Lead to the Development of Surrogate Tests for Immunosuppressive Activity (Abstract 15)
Renée Bazin

Plenary Session 3 – Exosomes

Friday, May 4, 8:45-10:15 ***Plenary Hall***

Chair: Daniel J. Weiss

Extracellular Vesicles (EVs) and other particles released by cells are increasingly recognized as a means of cell to cell communication and a powerful means by which physiologic and disease processes are mediated. However, the field is still in relative infancy and there remain many basic questions about particle identity, isolation and purification techniques, overall homeostasis, and potential use in therapeutics. These topics will be explored by three leading international experts in EV biology.

Manufacturing and Characterization of MSC-EVs for Clinical Testing

Eva Rohde

Extracellular vesicles (EVs) derived from stem and progenitor cells may have therapeutic effects comparable to their parental cells and are considered promising agents for the treatment of a variety of diseases. Strategies are needed to successfully translate EV research and to develop safe and efficacious therapies.

Based on the experience in manufacturing biological therapeutics for transfusions or for clinical studies, the presentation will focus on the development of mesenchymal stromal cell (MSC)-derived EV-based therapeutics. MSC-EVs are currently under investigation for their immunomodulatory, neuroprotective and tissue regenerative potential.

The requirements for manufacturing, safety, and efficacy testing of MSC-EVs during the translational development are comparable to those for various biologicals. The anticipated heterogeneity and complex molecular structure of EV-therapeutics may require thoroughly defined procedures and standardization of production will be a major issue. The mode of action (MoA) of putative EV-therapeutics will depend on their parental cells and will vary with the intended use for specific target diseases.

While the scientific community, pharmaceutical companies and clinicians are at the point of entering into clinical trials to test various EV-based therapeutics, the identification of the MoA underlying a suggested potency in each therapeutic approach remains a major challenge to the translational path.

Exosomes- CAP-2003

Linda Marban

Capricor Therapeutics is developing CAP-2003, an exosome product, for use in diseases of inflammation and fibrosis. Exosomes are nanometer sized vesicles that serve as cellular messengers. They were discovered over thirty years ago and were originally thought to be involved in cellular waste removal. Since then, exosomes have slowly risen to the fore of scientific curiosity as mediators of everything from metastasis to tissue regeneration. The exosomes in development by Capricor are proprietary to their cell source, the CDC, and have been shown to be markedly immunomodulatory as well as anti-fibrotic and pro-regenerative. Exosomes are a novel way to achieve cellular repair without the living material of the cell. The first indication will be to treat HLHS, otherwise known as hypoplastic left heart syndrome. HLHS is a congenital anomaly of the heart that requires immediate surgical repair at birth followed by two other surgeries during childhood. Exosomes delivered at the time of surgery may delay time to transplant as most of these children develop heart failure and require transplant relatively early in life. Process development of an exosome product will also be discussed.

MSC Exosomes and Small EVs: a Comparative Review

Sai-Kiang Lim

The rationale for the therapeutic use of MSCs has been increasingly attributed to their secretion, in particular exosomes or small extracellular vesicles (EVs). The term “small MSC EVs” generally refers to 50-200 nm EVs and is often used interchangeably with the term “exosomes” which refers to similarly sized EVs that are derived from endosomes. in practice, most MSC exosomes are purified by size and not by properties unique to exosomes. Hence most MSC exosome preparations are at best exosome-enriched small EV preparations, and by extrapolation, most small MSC EV preparations despite having a narrow size distribution are heterogenous. This presentation will review the degree of heterogeneity in small MSC EV preparations and the challenges that it poses to understanding MSC exosome biology and applications.

Plenary Breakout 1 – Exosome Technology

Friday, May 4, 10:45-12:15 ***Room 518A***

Chair: Eva Rohde

Exosome Engineering for Delivery of Therapeutic Proteins: Principles and Applications

Chulhee Choi

- To understand the potential of exosomes as novel biomarkers and therapeutics
- To understand the advantages and disadvantages of exosome-based delivery of drugs or biopharmaceuticals
- To understand the limitations of exosome-based therapeutics

Production of Functionally Bioactive EVs from an Immortalized Human Neural Stem Cell Line and their Therapeutic Application

Randolph Corteling

To ensure the scale required for clinical research and development, producer cell immortalization and clonal isolation is a practical strategy to produce consistent, functionally bioactive exosomes for use as therapeutic agents. Using the conditioned media produced during GMP manufacture, we have shown that the cell line is an abundant producer of exosomes which can be readily isolated, purified at scale and has demonstrated efficacy in a number of pre-clinical models.

ORAL ABSTRACT PRESENTATIONS:

Licensing Increases the Quantity and Immunomodulatory Cargo of Mesenchymal Stromal Cell Exosomes (Abstract 22)
Anastasia Cheng

NK Cell Expansion With Ex21-Exosomes (Abstract 23)
Jeremiah Oyer

Plenary Breakout 1 – Pre-Clinical Application of Exosomes

Friday, May 4, 15:30-17:00 ***Room 519AB***

Chair: Linda Marban

Heterogeneity of MSCs and Clinical Potential of their Extracellular Vesicles

Lambros Kordelas

- Delineate heterogeneity of MSCs
- Report on first application of MSC-derived EV in a case of steroid-refractory GvHD
- Highlight current research activities and possible future applications

Apoptotic Exosomes: New Players in the Regulation of the Immune Response

Marie-Josée Hébert

- Demonstrate the difference between apoptotic bodies and apoptotic exosomes on the immune response
- Describe the importance of apoptotic exosomes in accelerating allograft rejection
- Illustrate the importance of apoptotic exosomes in activating auto-immune responses

ORAL ABSTRACT PRESENTATIONS:

The X-Factor Involved in Apoptosis and Proliferation – Reciprocal Exosome Cross-Talk Between Lymphangioleiomyomatosis-Derived Smooth Muscle Cells (LAM-SMCs) and Endothelial Cells (ECs) Mediates EC Network Disruption/Apoptosis and Promotes LAM-SMC Growth (Abstract 24)
Mirabelle Ho

MSC Exosomes Alleviate Pain and Degeneration in a Rat Model of Temporomandibular Joint Osteoarthritis (Abstract 25)
Wei Seong Toh

Plenary Session 4 – Reimagining Cancer Care and Delivering on the Promise of CAR-T Therapies

Friday, May 4, 13:45-15:15 ***Plenary Hall***

The session will cover the recent developments and success with CAR-T therapies.

The vision of translation from academia to industrial development and eventual market launch will be discussed focussed on the recent market launches. This will represent an opportunity to give the audience, both the academics as well as the industry, to hear and discuss the different angles and models that lead to the CAR-T success stories. These examples will be looked with an aim to the future as lessons learned and the impact on the field of cell and gene therapy and the commitment of different stakeholders to this emerging value adding immunotherapy approach to unmet medical needs.

CAR T: Current Status and Perspectives form the Academic Lens

Carl June

Reimagining Cancer Care and Delivering Kymriah to Patients

Pascal Touchon

Making History – Commercial Launch of CAR-T Therapy

Diane Parks

This talk will cover how Kite moved from the clinic to the commercial launch of Yescarta (axicabtagene ciloleucel). Building the market, ensuring access and delivering the product were key strategic imperatives that required unique strategies and flawless execution. This talk will provide a look back on the road to commercialization and insight into some things others contemplating moving into this space should consider.

Plenary Breakout 2 – Lessons Learned During CAR T Commercial Roll Out at Clinical Sites

Friday, May 4, 10:45-12:15 ***Room 520CF***

Chair: Sarah Nikiforow

While CAR T research has been ongoing for over 20 years, the last few years have seen an explosion of constructs, products, sponsors, trials and number of patients and clinical sites involved in the effort. With the transition from clinical trial products and distribution to commercial approval and dispensation under a REMS program, even experienced sponsors/manufacturers and academic clinical sites have had to modify workflows and resources to meet new demands. in this session, our speakers will focus on several lessons learned in the transition process. For commercial manufacturers, this has included training clinical sites on safety and patient selection outside of previously explicit/prescriptive clinical trial designs and discussion of resources needed for REMS program compliance; education on unique apheresis and distribution workflows and interfaces; and creating different contracting models. For clinical sites, this has mandated an evaluation of multiple resources to support a safe and effective cell therapy program of higher volume than prior, outside of the typical clinical trial infrastructure. Differences between global and US as well as pediatric and adult approaches will be briefly touched upon.

Preparing Sites to Enter the Commercial CAR T Setting: Focus on Clinical Readiness

Solveig Ericson

Best Practices – Supporting Logistical Accounts Transitions for Commercial CAR T Distribution

Diane Parks

Growing Pains in an Academic CAR T Program, Including Transition from Clinical Trials to Commercial Products

Sarah Nikiforow

Plenary Breakout 2 – Market Access & Reimbursement of CAR T Therapies

Friday, May 4, 15:30-17:00 ***Room 520CF***

Chair: Dawn Driscoll

This session will expand upon the concepts of Plenary Session 4, Reimagining Cancer Care and Delivering on the Promise of CAR-T Therapies, and we will take a deeper look at multiple aspects of market access for advanced cellular immunotherapies, using CAR-T therapies as our example. While these new therapies offer unprecedented clinical outcomes, they are costly to patients and health care systems. Our speakers will examine these complex issues and discuss experiences in their countries and organizations. Each speaker will present for 15 minutes, and then we will have an interactive panel Q&A session with the audience.

Market Access Learning from the First CAR-T in the Adult Population

Rocio Manghani

- Identify key best practices in the launch of CAR-T for adult patients with relapse/refractory large B-cell lymphoma.

CAR-T Therapy, a Patient's Perspective

Douglas Olson

Valuing Cellular, Regenerative, and Gene Therapies: The Intersection of Promise, Uncertainty, and Cost

Daniel Ollendorf

- To summarize traditional approaches for valuing new health technologies through cost-effectiveness and budget impact analysis
- To describe the ways in which new cell/gene therapies with curative potential turn traditional value analysis on its head
- Through case examples, to describe approaches that can address uncertainty in data, manage costs over the short- and long-term, and ensure access for highest-priority patients

Implementing Gene Therapies in the Canadian Health System: What Does the Future Look Like?

Tammy Clifford

Delegates will:

- Understand current regulatory and reimbursement approaches
- Appreciate the unique opportunities and challenges facing the introduction of gene therapies in the Canadian health system
- Recognize the current & future needs of decision makers

Plenary Session 5 – ISCT-ASGCT Joint Session: Genome Editing for Benign and Malignant Diseases

Saturday, May 5, 9:15-10:45 ***Plenary Hall***

Co-Chairs: Donald Kohn and Helen Heslop

In this joint session hosted by ISCT and ASGCT on Genome Editing for Benign & Malignant Diseases, recent advances in gene editing will be reviewed.

Presentations will include: Ex Vivo Gene Modification of HSC by Matthew Porteus (Stanford University); ZFN Targeting of CCR5 in HSC for HIV by John Zaia (City of Hope Medical Center); and in Vivo Targeting of Liver by Michael Homes (Sangamo Biotherapeutics). The goal of the session is to provide attendees with the latest information on the approaches and status of genome editing.

Gene Editing of Hematopoietic Stem and Progenitor Cells

Matthew Porteus

Genome editing provides a method to precisely change the DNA sequence of a cell, including somatic stem cells. We have developed a system that combines the use of CRISPR/Cas9 delivered as ribonucleoprotein complex with AAV6 transduction to generate high frequencies of genome editing in a wide variety of human cell types including hematopoietic stem cells, T-cells, mesenchymal stromal cells, airway basal stem cells, neural stem cells and pluripotent cells. in this presentation I will discuss our application of this system to engineering human cells to treat human disease.

Genome Editing with ZFN using Blood Progenitor Cells in HIV/AIDS

John Zaia

Gene therapy and genome editing are modalities with application to HIV/AIDS, and this presentation will review the current state of the field for this area of clinical research. The speaker will then focus on the feasibility issues regarding scale-up for ZFN-based genome editing of hematopoietic stem/progenitor (HSPC) cell transplantation. The first clinical trial of CCR5-targeted ZFN-modified HSPC is being performed in HIV/AIDS patients, and the laboratory and regulatory hurdles required for initiation of this first-in-human trial will be reviewed. This active outpatient study has now treated 7 subjects, and selected preliminary results will be reviewed. All patients have engrafted with CCR5-disrupted cells and the conditioning regimen, utilizing busulfan, was well tolerated in these HIV-infected subjects.

Highly Efficient and Specific Multiplexed Gene Editing in T Cells Using Enhanced Zinc-finger Nucleases (ZFNs) Enables Strategic Engineering of Allogeneic T Cell Immunotherapies

Michael Holmes

Engineered allogeneic CAR-T cells generated from healthy donors would provide a well-characterized, consistent off-the-shelf product that could be administered to a broad population of patients. to pursue this approach, a highly efficient and precise gene editing capability is imperative to generate the desired T cell product profile: TCR/CD3-negative achieved by knocking out T-cell receptor alpha constant region (TRAC), HLA class I-negative achieved by disrupting HLA complex formation by knocking out the β2-microglobulin (β2M) gene, and positive expression of an antigen-specific CAR or TCR achieved by site-directed gene insertion into the disrupted TRAC locus.

Designed ZFNs provide an attractive platform for therapeutic genome editing, as they can be designed to target virtually any site in the genome with a high degree of precision, efficiency, and specificity. Using ZFNs, we have developed multiplexed gene editing capabilities that can simultaneously disrupt the TRAC and β2M loci at a double knock-out (KO) efficiency of >90%, and achieve targeted insertion of a transgene into TRAC at >90%. Furthermore, a rigorous and deep molecular interrogation of the ZFN-treated T cells revealed undetectable off-target nuclease activity, highlighting the specificity of our enhanced ZFN technology. These industry leading capabilities enable the development of defined and well-characterized allogeneic CAR-T cell products for application in hematological malignancies.

Plenary Breakout 1 – Editing to Enhance Cell Therapy – Universal T Cells

Saturday, May 5, 11:00-12:00 ***Room 519AB***

Chair: Helen Heslop

Engineering Regulatory T Cells for Inflammatory Disorders

Andrew Scharenberg

- Understand the basics of regulatory T-cell biology
- Understand how gene editing tools are applied for engineering regulatory-phenotype T-cells
- Understand how synthetic biology intersects with gene editing to allow generation of novel cell properties

ORAL ABSTRACT PRESENTATIONS:

Single Cell Analysis of Lentiviral Integration to Support Ex-Vivo Gene Modified Cell Therapy Development (Abstract 29)
Vincenzo Di Cerbo

CRISPR-Driven Modeling of Clinically Relevant Genetic Variants in Hematopoietic Stem and Progenitor-Derived Erythroid Cells (Abstract 369)
Yelena Boccacci

Plenary Breakout 2 – Ex Vivo Editing – HSC and iPSC

Saturday, May 5, 11:00-12:00 ***Room 520CF***

Chair: Matthew Porteus

Improving Homology-Directed Repair in Hematopoietic Stem Cells

Donald Kohn

- Be familiar with methods to deliver homologous donors to HSC.
- Understand the role of HSC cell cycle status on homology-directed repair of genes.
- Appreciate the current translational status of gene editing to clinical trials

ORAL ABSTRACT PRESENTATIONS:

How Genetically Modified Organism (GMO) Regulations Impact On The Development of Genetically Modified Cell-Based Therapies in Europe, The U.S. and Japan (Abstract 31)
Houria Bachtarzi

Adoptive Transfer of Genetically Engineered Monocytes for the Tumour Targeted Delivery of IFN-Alpha (Abstract 32)
Roberta Mazzieri

Plenary Session 6 – iPSC Regenerative Medicine

Saturday, May 5, 13:30-15:00 ***Plenary Hall***

Chair: Andras Nagy

Translating Stem Cell Biology into Regenerative Medicine

Maria Mirotsou

Pluripotent Stem Cells (PSCs) hold great promise for drug discovery and regenerative medicine. Despite the complexity, major advances over the last decade have resulted in reproducible protocols that generate well-defined cells and a number of clinical trials testing PSC-derived products in humans. However, difficulties in proper cell maturation, integration and survival still exist. My presentation will focus on these challenges, especially in progressing from early scientific concepts to product development, and provide an overview of the opportunities for innovation in the field.

Making iPSC Cell Derived Products

Mahendra Rao

The discovery of induced pluripotent stem cells (iPSCs) and the ability to manufacture them using clinically compliant protocols has the potential to revolutionize the field of regenerative medicine. However, realizing this potential requires developing processes that are reliable, reproducible, and cost-effective that at the same time do not compromise the safety of individuals receiving this therapy. in this presentation we discuss the ways cost reduction can be obtained using real world examples. These include modest changes in regulation, developing DMF's, nd generating reference material and stable reporter lines.

One important cost reduction strategy that I will emphasize is to take advantage of the inherent immortality of iPSC and show that multiple cellular products currently being considered for therapy can be generated from a single master cell bank of any clinically compliant iPSC line. a second major cost reduction strategy is to reduce the cost of preclinical studies to do we introduced a constitutively expressed GFP cassette in Chr13 safe harbor site using a standardized previously described method We believe that our demonstration that multiple products can be made from the same WCB, and that the same protocols can be used with multiple lines offers the potential of making iPSC cell based therapy affordable.

A Solution for Cell Therapy Safety and Engineered Allotolerance

Andras Nagy

Pluripotent stem cells have accelerated the development of new avenues for targeting degenerative diseases. Numerous cell therapies are currently on their way to treat devastating conditions. However, concerns about the cell-safety hold back the full utilisation of these promising new treatments. Here I introduce a concept and show the associated genome engineering strategy that addresses this issue and provides a solution for "fail-safe" cell therapies.

Using published and our experimental measures, we defined mathematically the level of safety of therapeutic batches of cells. Our general approach to assess and quantify the safety will be critical to make informed decisions by the regulators, doctors, and patients to advance this modern medicine-transforming therapies.

Building on the fail-safe technology, we addressed the next hurdle faced by cell therapies; a solution for induced allograft tolerance. I show that the expression of eight local-acting,

immunomodulatory transgenes introduced into embryonic stem cells is sufficient to protect cell derivatives against rejection in allogenic, immune-competent recipients. Allografts survive long-term, in different MHC-mismatched recipients, and without immunosuppressive drugs.

The combination of the fail-safe and immune tolerance genome editing will make the "One for All" cell line and therapeutic cell development a reality.

Plenary Breakout 1– iPSC and Organogenesis

Saturday, May 5, 15:15-16:30 ***Room 519AB***

Chair: Maria Mirotsou

Making Functional Islet Cells from Human Pluripotent Stem Cells

Timothy Kieffer

- Define the rationale for a cell based therapy for diabetes
- Describe strategies to produce islet cells from stem cells
- Assess the impact of stem cell differentiation state on outcomes following implant

Modeling Human Retinal Degenerative Diseases Using iPSC-derived 3D Photoreceptor Sheets

Gilbert Bernier

- Development of a method to generate 3D cone photoreceptor sheets from hESCs
- Application of the method to produce 3D cone photoreceptor sheets from patient-derived iPSCs
- Next-generation RNA-seq to better understand the disease's mechanisms

ORAL ABSTRACT PRESENTATIONS:

Rapid and Robust Cd4+ and Cd8+ T-, Nk-, B- and Monocyte Cell Reconstitution After Nicotinamide-expanded Cord Blood (nicord) Transplantation (Abstract 37)
Jaap Jan Boelens

Plenary Breakout 2 – iPS Generation and Skin and Lung Applications

Saturday, May 5, 15:15-16:30 ***Room 520CF***

Chair: Mahendra Rao

Collagen VII Skin Grafts for Chronic RDEB Wounds

Jean Tang

This presentation will review the 15 years of pre-clinical, regulatory and clinical development of LEAES/EB-101 keratinocyte skin grafts for chronic wounds in patients with Recessive Dystrophic Epidermolysis Bullosa (RDEB). Autologous keratinocyte skin sheets are transduced with a retrovirus to express Collagen 7, the defective protein in RDEB. Six gene corrected skin grafts are generated from two small biopsies, and are currently being manufactured at the LCGM/GMP facility for a Phase 3 clinical trial in adults and children with RDEB.

ORAL ABSTRACT PRESENTATIONS:

Safety Considerations in the Generation of Clinical Grade Autologous iPS Cell Lines (Abstract 38)
Holly Anderson

Close Neighbors in the Niche: Paracrine Signals from Endothelial Cells Promote Alveolar Epithelial Differentiation of Induced Pluripotent Stem Cell-derived Lung Progenitors (Abstract 39)
Miriel Ho

Allogeneic Amniotic Epithelial Cells for Established Bronchopulmonary Dysplasia in Premature, Low Birthweight Infants: a first-in-human Safety Trial (Abstract 40)
Rebecca Lim

Hot Topic Sessions

Hot Topic Session 1 – Tregs and Other Cell Subtypes/Immunotherapy in Autoimmune Disease

Thursday, May 3, 7:30-8:30 ***Room 520BE***

Chair: David DiGiusto

The use of regulatory T-cells to combat autoimmunity and GVHD and other (mesenchymal) forms of stem cells for immune-based disease indications is an area of significant interest to ISCT investigators. in this session the presenters will discuss the identification, pre-clinical evaluation and clinical implementation of various forms of regulatory T-cells in hematopoietic stem cell transplantation and eASC in Crohn's disease.

Everett Meyer

The Emerging Role of Cell Therapies in Gastrointestinal Disorders

Alan Burns

Gastrointestinal disorders comprise a wide range of often difficult to manage conditions with few definitive cures.

- Regenerative medicine approaches, such as cell therapies, offer novel ways to repair the gastrointestinal tract.
- The use of mesenchymal stem cells (MSCs) to treat perianal fistulas will be outlined.

- The idea of using enteric neural stem cells (ENSCs) for enteric neuropathies will be discussed.

Hot Topic Session 2 – Approaches to Overcoming Limitations of Cell and Gene Therapies

Thursday, May 3, 7:30-8:30 ***Room 520CF***

Chair: Sandeep Soni

Transduction Efficience is a Critical Component Defining the Clinical Outcome in Gene Therapy

Gabor Veres

- Experimental strategies to increase human hematopoietic stem cell transduction for clinical applications based on disease indications
- Initial experience in clinical setting in Thalassemia and Sickle cell disease, transduction efficiency and clinical outcome
- How improvement in transduction efficience can translate into better clinical outcome

Antibody-based Conditioning for Hematopoeitic Stem Cell Therapies

Judith Shizuru

- Describe the use of antibodies directed against CD117 to create stem cell niche space and allow engraftment of hematopoietic stem cells.
- Discuss novel ways to potentiate the cell depletive effect of anti-CD117 antibodies
- Present early results of a clinical trial using anti-CD117 antibody as sole conditioning for patients with severe combined immune deficiency.

Programmed Cellular Immunotherapies for Fighting Cancer

Daniel Shoemaker

- The phenotypes / biological properties of cellular therapies can have a significant impact on patient outcomes (better cells = better therapies).
- The therapeutic properties of cellular therapies can be enhanced by ex vivo modulation with small molecules.
- Off-the-shelf iPSC derived NK and T-cell products have the potential to overcome many of the challenges facing autologous cell therapies (e.g., CAR-T).

Hot Topic Session 3 – Immunologic Sculpting of T Cell and Stem Cell Grafts

Friday, May 4, 7:30-8:30 ***Room 520BE***

Chair: Denis-Claude Roy

Can we generate cells with anti-infection and anti-leukemia activity without triggering graft-vs-host disease or other side effects? This session will cover how manipulation of the stem cell graft and addition of specifically selected or expanded T cells can help overcome these issues. Novel developments in the use of CAR T cells as alternative or adjunct to eliminate malignant cells will also be discussed.

Haploidentical Transplant in the Era of Graft Engineering: What's New in Pediatrics

Alice Bertaina

- T-cell depleted haploidentical transplant represents an alternative treatment for children lacking a matched-sibling donor
- Thanks to new approaches of graft manipulation, T-cell depleted haploidentical transplant is a valid option not only for leukemia patients, but also for children with several non-malignant disorders
- alfa/beta T-cell/CD19+ B-cell depletion translates in high rate of cure with low risk of both acute and chronic GvHD in children with acute leukemia

Anti-infection and Anti-leukemia Donor T cells Without GVHD: "Mission Possible"

Denis-Claude Roy

- Understand mechanisms leading to selective elimination of alloreactive T cells.
- Learn about minor histocompatibility antigens and how they can be used in the context of cellular immunotherapy.
- Gain insights into clinical implementation and results of T cell selection and expansion strategies in the context of HLA-matched and haplo-identical stem cell transplantation.

Advances in CAR T Cell Therapy: the CD19 CAR Paradigm

Isabelle Rivière

- Discuss the success and challenges of CAR T cell therapies
- New approaches to increase CAR T cell therapy efficiency

Hot Topic Session 4 – Oncolytic Viruses

Friday, May 4, 7:30-8:30 ***Room 520CF***

Chair: John Bell

Oncolytic or cancer lysing viruses have been engineered or selected to replicate in and kill cancer cells while leaving normal cells unaffected. in this session some of the latest pre-clinical and clinical advancements in the field will be discussed.

Development of a Novel Rhabdovirus Platform for the Treatment of Cancer

John Bell

Oncolytic Virus Engineered Cell Based Therapeutics for Cancer

Khalid Shah

- Fate of OV loaded stem cells and OV mediated tumor cell killing
- Efficacy of engineered stem cells in different tumor models
- Clinical translation of OV engineered stem cells

ORAL ABSTRACT PRESENTATION:

Functional Annotation and Identification of Putative Drug Target in Strain Ankara (Abstract 21)
Yashbir Singh

Hot Topic Session 5 – Solid Organ Transplantation: Tolerance Induction and Tregs Immunotherapy

Saturday, May 5, 8:00-9:00 ***Room 519AB***

Chair: Stephan Busque

This session will explore the intersection between solid organ transplantation and cell therapy that is at the basis of tolerance induction after solid organ transplantation. The experience from 2 leading programs in this field (Stanford and Northwestern) will be presented. The potential benefits of using Tregs as immunotherapy after kidney transplant to favor tolerance will also be discussed.

Tolerance Induction After Kidney Transplantation: Overview and Stanford's Experience

Stephan Busque

- Identify the potential benefits of tolerance Vs conventional chronic immunosuppressive therapies
- Recognize that the risk of GVHD needs to be minimal in this patient population
- Compare the chance of success to be tolerant between matched and miss-matched pairs

Therapeutic Cell Transfer to Achieve Transplantation Tolerance: from Facilitating Cells to Regulatory T cells

Joseph Leventhal

- Understand the importance of tolerance to potentially improving outcomes in solid organ transplantation
- Review the current status of engineered stem cell, so called Facilitating cell based approach to achieving tolerance in kidney transplantation
- Review the use of regulatory T cell based therapies in solid organ transplantation conducted at Northwestern University

Hot Topic Session 6 – Biomaterials and Clinical Uses

Saturday, May 5, 8:00-9:00 ***Room 520CF***

Chair: May Griffith

Biomaterials are now being used to enhance cellular therapeutics. These are most widely used as nanoparticles for delivery systems for drugs and bioactives, and also in diagnostics. More recently biomaterials are being developed as scaffolds for exogenous and endogenous stem cells to affect regeneration. This aim of this session is to highlight a few examples of biomaterials designed to enhanced cellular therapeutics in regenerative medicine.

Biomimetic Materials and Cornea Regeneration: from Bench to Bedside and Back

May Griffith

The objectives of this presentation are to show the journey from designing biomaterials, fabrication, pre-clinical and translational manufacturing protocols following GMP to clinical evaluation in a first-in-human trial to regenerate the human cornea as an alternative to allografting. Here biomaterials-based implants stimulate the patients' endogenous stem cells to regenerate neo-corneas. I also show how incremental changes to a biomimetic scaffold can be made to adapt it to a patient population at high risk for rejecting conventional donor tissue allografting, or for the potential extension to other organs systems.

Tissue Engineered Corneal Epithelium for Clinical Application

Lucie Germain

- Learn about stem cells of the cornea.
- Understand the process of tissue engineering an autologous epithelium.
- Learn about the translation from experimental to clinical study.

Biomaterials for Engineering of Bone

Jöns Hilborn

- Provide design criteria for regenerative matrix gel materials
- How to collaborate for success
- Identify cost effective therapies

Biomaterials to Translate Promising Therapies for Neural Regeneration to Humans

Eve Tsai

- Participants will learn that biomaterials are promising to translate promising therapies to regenerate the brain and spinal cord from animals to humans.
- Participants will learn why biomaterials may be necessary to translate promising therapies to regeneration the brain and spinal cord from animals to humans.
- Participants will learn the important areas for further knowledge, development, and research with respect to brain and spinal cord regeneration and repair.

Advanced Practice Professionals (APP) Track Sessions

Advanced Practice Professionals (APP) Track Session 1 – Pharmacy Roles in Cellular Therapy & Hematopoietic Stem Cell Transplant

Thursday, May 3, 11:00-12:15 ***Room 518A***

Chair: Tobi Fisher

Old Drug-New Tricks: Post-transplant Cyclophosphamide in HSCT Setting: Key Opportunities for Clinical Pharmacists

Rebecca Gonzalez

- Describe clinical issues with expanded use of post-transplant cyclophosphamide (PTCy)
- Explore adverse effects and management of immunosuppressive regimens with PTCy
- Discuss pharmacists role in counseling and collaborative management of HSCT patients

Racing Towards the Finish Line with CAR-T cell Therapy: The Pharmacist's Role in the Pit Crew

Christina Bachmeier

- Identify operational considerations associated with implementing chimeric antigen receptor (CAR) T-cell products into clinical practice
- Describe the management of adverse effects associated with CAR-T cell therapy
- Discuss the pharmacist's role in counseling and collaborative management of CAR-T cell patients

Advanced Practice Professionals (APP) Track Session 2 – CAR T Immunotherapy: Science and Logistics

Thursday, May 3, 15:45-17:00 ***Room 518A***

Chair: Megan Cornelison

Supporting Investigator Initiated Trials

Bambi Grilley

- Provide practical considerations for assisting in the development and implementation of IIT interventional studies
- Discuss when an IND is required, the various types of INDs, and the content of an IND submission
- Present other required processes and documents that may be required or can be helpful in the conduct of IIT IND studies

Understanding and Managing Toxicities of CAR T cells

Stanley Riddell

- Identify clinical signs of cytokine release syndrome and neurotoxicity
- Current understanding of the pathogenesis of toxicities of CAR T cells
- Evolving management of short and potential long term toxicities of CAR T cells

Advanced Practice Professionals (APP) Track Session 3 – What's New in Cord Blood Transplantation for Hematologic Malignancies

Thursday, May 3, 17:00 – 18:30 ***Room 518A***

Chair: Tobi Fisher

Intermediate Intensity Double Unit CBT as a Platform for Cellular Therapy

Juliet Barker

- To understand the value of unrelated donor cord blood transplantation (CBT) to extend allograft access to minority patients.
- To understand that high progression-free survival can be achieved after intermediate intensity double unit CBT in adults with hematologic malignancies.
- To understand that intermediate intensity CBT is a platform for the application of new cellular therapy approaches.

CBT: Day to Day Management of CBT Recipients: Graft Infusion and Specialized Pharmacology

Valkal Bhatt

Additional Session Summaries

Scientific Area of Focus – Stem Cell Biology/ Expansion

Thursday, May 3, 15:45-17:00 ***Room 520AD***

Chair: John Rasko

Universal Donor Cord Blood Cell Therapies: Expanding Clinical Access and Improving Clinical Feasibility

Colleen Delaney

- Discuss the role of umbilical cord blood as a source of donor stem cells for hematopoietic stem cell transplant, including the procs and cons of this stem cell source and strategies to improve outcomes.
- Discuss the development of a universal donor stem/ progenitor cell for clinical application in hematologic malignancies.
- Discuss the logistics of implementing expanded progenitor cell therapies in clinical practice.

Cellular Engineering with UM171: From Bedside to Benchtop

Guy Sauvageau

- Understand the need for cord blood engineering
- Understand the development of UM171 from bench to clinics
- Show results from clinics to bench

Vascular Niche Signals for Expansion of Engraftable Stem Cells

Shahin Rafii

Objectives:

- Demonstrate that organ-specific vascular niche by deploying angiocrine factors drive the self-renewal of true repopulating stem cells.
- Master cells banks of engineered vascular niche endothelial cells have been manufactured for clinical expansion of cord blood, bone marrow and mobilized peripheral blood hematopoietic stem cells.
- Clinical trials planned for first-in-human co-transplantation of endothelial and expanded stem cells.

Scientific Area of Focus – NK Cell Therapy: a New Frontier Revisited

Friday, May 4, 10:45-12:15 ***Room 520AD***

Chair: Armand Keating

Novel Strategies to Activate NK Cells to Treat Cancer

Sarah Cooley

Armored CAR NK Cells for the Treatment of Lymphoid Malignancies

Katy Rezvani

- Discuss strategies for the ex vivo expansion of NK cells for the treatment of cancer
- Present data on the first-in-human trial of off-the-shelf armored CAR-transduced NK cells in lymphoid malignancies

Cell Therapy with Permanent NK Cell Lines

Armand Keating

- Understand the rationale for the use of permanent NK cell lines in the treatment of cancer.
- Appreciate the advantages and limitations of these NK cells in treating cancers.
- Review the strategies and underlying mechanisms available to enhance the cytotoxicity of NK cell lines against a variety of tumor types and compare them with T cell based cellular immunotherapies

ISCT Presidential Task Force Session – Tailoring Communication on Cell Therapies Between Scientific Societies, Regulations, Bioethical Needs and Patient Perspectives

Friday, May 4, 10:45-12:15 ***Room 519AB***

Chair: Massimo Dominici

The ISCT Presidential Task Force (PTF) on the Use of Unproven Cellular Therapies (UCT) is a prominent group of academic, industry, bioethical and regulatory experts in the cell therapy field. This session is focused on understanding patient-focused communications surrounding unproven cellular therapies and the role of scientific/medical societies, health-care stakeholders, and regulators to promote rigorous research and development of safe and effective cell therapies. The PTF-UCT will share the results of a global collaborative survey that involved many scientific societies and organizations on how UCT are impacting several fields of biomedical research and clinical practice. Representatives from FDA and Health Canada – two key regulatory bodies -- will then deliver presentations on current regulatory activities

also related to unapproved, and potentially unethical, cellular therapies. This session aims to integrate considerations of regulatory issues with patients' needs. For this reason it will also feature a physician perspective and a patient success story from a regulated clinical trial in a neurological condition, such as multiple sclerosis. Finally, relevant bioethical considerations, focusing on communication practices among various cell therapy stakeholders, will be discussed to highlight how ethical considerations can be integrated into the development of cell-based therapeutics as part of a patient-centric unified process.

Massimo Dominici

FDA Perspectives on Stem Cell Therapies

Peter Marks

- Describe the FDA's regenerative medicine framework
- Discuss the Agency's efforts to facilitate development of novel stem cell products
- Discuss the steps the Agency is taking toward ensuring compliance with regulations.

Regulation of Autologous Cell Therapies in Canada

Nadine Kolas

To provide:

- An overview on the regulatory landscape of cell therapies in Canada
- Information on policy considerations related to autologous cell therapies in Canada
- Information on the regulation of autologous cell therapies in Canada

A Physician's Perspective on Unproven Cell Therapies: How Hope Has Become a Commodity

Harold Atkins

- Understand the need for transparent and detailed information about cell therapy products.
- Describe some of the barriers experienced in the knowledge translation of new and developing cell therapies and how this might influence a patient's decision to undergo an unproven stem cell treatment.
- Explore how marketing "hope" as a commodity contributes to the growth of unproven cell therapies.

Ethical Considerations in Communication Surrounding Cellular Therapies

Aaron Levine

- Articulate ethical issues relevant to communicating with multiple stakeholders about cellular therapies
- Identify potential pitfalls associated with communicating about cellular therapies
- Describe preliminary best practices to improve communication surrounding cellular therapies

Non Myeloblative HSCT: A Patient's Perspective

Keith Moore

Early Stage Professionals Session 1 – Translating Cell and Gene Therapy Products from Bench to Bedside

Friday, May 4, 15:30-17:00 ***Room 518A***

Co-Chairs: Patrick Hanley and Fernanda Masri

The objective of this session will be to take a practical look at how key stakeholders play a role in translating a cell or gene therapy product from the bench to the bedside. This includes perspectives from a basic scientist, a GMP facility director, a regulatory director, and an investigator involved in translating the therapy to a biotech company.

Cell Therapy Translation – Pathways and Pitfalls

Kenneth Micklethwaite

- Describe the steps required to translate new therapies to clinical trials
- Identify pitfalls in the development of new cellular therapy products
- Identify resources for young investigators trying to translate new cellular therapy products into the clinic

Paul Eldridge

Developing and Implementing Investigator Initiated Trials: Practical Considerations

Bambi Grilley

- Provide practical considerations for developing and implementing successful IIT interventional studies
- Discuss when an IND is required, the various types of INDs, and the content of an IND submission
- Discuss Good Clinical Practice as it applies to the conduct of IIT IND studies

The Path From Lab Bench Discovery to Startup Company

Sadik Kassim

- To discuss the challenges and opportunities of transitioning from lab bench discovery to startup company in the cell therapy space.
- To discuss the challenges and opportunities in transitioning a cell therapy manufacturing process from the academic to the industrial setting.
- To discuss other issues associated with building a startup based on an academic based discovery, including tech transfer, licensing, and IP issues.

Abstract Showcase Sessions

ISCT Chief Scientific Officer Abstract Showcase

Thursday, May 3, 15:45-17:00 ***Room 518BC***

Chair: Daniel J. Weiss

ORAL ABSTRACT PRESENTATIONS:

Identification of Differential Expression Phenotypes of CD133+ Stem Cells in Acute and Chronic Myocardial Infarct Patients and Specific Expression Pathways Underpinning Therapeutic Responsiveness in Regenerative Therapy (Abstract 16)
Shant Der Sarkissian

Developing of Tissue Engineered Auricular Pavilion Able to Induce Host Perichondrium and Subdermal Tissue Integration Preclinical Feasibility and Safety Study (Abstract 17)
Gustavo Moviglia

Large-Scale, Single Cell RNA Sequencing Defines Novel Cellular Subsets Required for Cardiac Repair (Abstract 18)
Robert Nordon

Designing Microtissue Bioassemblies for Skeletal Regeneration: Healing Critical Size Long Bone Defects (Abstract 19)
Ioannis Papantoniou

Combining Electrospinning and 3D Printing for a New Generation of Nerve Guides (Abstract 20)
Joel Simpson-Edin

ISCT 2018 Organizing Committee Abstract Showcase

Friday, May 4, 15:30-17:00 ***Room 520AD***

Co-Chairs: Sarah Nikiforow and Rachele Ciccocioppo

ORAL ABSTRACT PRESENTATIONS:

First Rapid Final Release Test of ATMPs Prior to Treatment – Validation of a Robust and Highly Sensitive qPCR Test for Total Bacteria (Abstract 26)
Kai Nesemann

Cardiosphere-Derived Cells Show Strong Immunomodulatory Activity and Improve Muscle Physiology When Systemically Delivered in a DMD Mouse Model (Abstract 27)
Jennifer Moseley

5-Year Performance Data and Robust Flexibility of a Computerized Physician Ordering System for Collection, Processing, and Administration of Cellular Therapy Products (Abstract 28)
Sarah Nikiforow

Direct Comparison of Adult Human and Rat Spinal Cord Stem Cell Behavior (Abstract 368)
Ahmad Galuta

Towards Gene-Editing Treatment For Alzheimer's Disease: ApoE4 Allele-Specific Knockout Using a CRISPR Cas9 Variant (Abstract 30)
Dani Offen

Expanding Cellular Therapies Through Provision of a Cord Blood – Derived iPSC Haplobank (Abstract 370)
Ngaire Elwood

Early Stage Professionals Session 2 – Young Investigator Abstract Showcase

Saturday, May 5, 11:00-12:00 ***Room 520AD***

Co-Chairs: Alireza Abazari and George Hucks

ORAL ABSTRACT PRESENTATIONS:

Safety of Stem Cell-Derived Encapsulated Liver Tissue to Treat Liver Failure: Immune-Isolation and Absence of Foreign Body Reaction or Tumor Formation Upon Transplantation Without Immunosuppression (Abstract 33)
Claudia Raggi

Combined Cell and Gene Therapy Towards The Treatment of Age-Related Macular Degeneration and Diabetic Retinopathy (Abstract 34)
Sabiha Hacibekiroglu

First Trimester Human Umbilical Cord Perivascular Cells (FTM HUCPVCs) Expanded With cGMP Compliant Human Platelet Outperform Conventional and Older MSC Sources as Regenerative Therapy in a Rat Myocardial Infarction (MI) Model (Abstract 35)
Alexander Johnston

In Vivo Tracking of CAR-T By [18F]BF4- PET/CT in Human Breast Cancer Xenografts Reveals Differences in CAR-T Tumour Retention (Abstract 36)
Ewelina Kurtys

How to Be Successful at Publishing your Work

John Barrett

- Learn the correct format for assembling a manuscript
- Understand what makes a manuscript likely to be published
- Learn successful rebuttal strategies

Quality and Operations Track Sessions

Quality and Operations Track Session 1 – Progress in the World of Standards for Cell and Gene Therapies

Thursday, May 3, 11:00-12:15 ***Room 520BE***

Chair: Sowmya Viswanathan

Session Objectives:

- Provide an understanding of why standards/reference materials are needed to accelerate cell and gene therapy products
- Provide an overview of standards development including activities from Standard Coordinating Body (SCB) and International Standards Organization (ISO)
- Provide more details on standard development from US, UK and Japan's perspective
- Provide an understanding of how voluntary consensus standards are being developed and adopted internationally

Standards Coordinating Body (SCB): Advancing Standards for Regenerative Medicines

Jiwen Zhang

The presentation will inform the audience on:

- The need of standards for the regenerative medicine field and standard development efforts initiated;
- The need of coordination in standard development efforts and the establishment of the SCB;
- SCB stake holders, strategic plans, activities, and projects including FDA projects;
- Call for stake holder collaborations to advance regenerative medicine standards.

Filling Information Gaps to Accelerate Commercialization: Two Case Studies in the Development of ISO Standards for Regenerative Medicine

Claudia Zylberberg

- Introduce the process by which two key ISO standards have been generated
  - Ancillary Materials (AM) standards and reference materials. Update on status and covered areas.
  - Transportation/Logistics. Update on status and covered areas - this will be from a supplier's perspective, complementing the Japanese presentation focused on part 1 of the standard
- Lessons from these two cases, and the future of standards in regenerative medicine
  - The ISO standards development process, focusing on the importance of expert consensus for alignment by relevant actors
  - Assessment of current efforts to address unmet needs, including an examination of other areas where standardization would support greater industry growth
- Emerging forums for standards development
  - Status and community deployment plan with SCB
  - Standards development and diffusion within ARMI and NIIMBL

Standards Activities For Manufacturing Characterisation and Control of Cell-based Products

Jonathan Campbell

Design for manufacture is critically important in the development of cell-based products due to them not being candidates for re-processing. Whist 'six-sigma process' adopts a control strategy based around Gaussian probability distribution, cell-based products may exhibit distinctly non-Gaussian behaviour due to the cell component being able to influence its own environment.

The features that influence manufacturability of cell-based products will be indicated and discussed, including inherent heterogeneity but also variation arising from behaviours of living entities where characterisation of only small parts of a cell is possible. Unless characterisation is sufficiently robust (preferably demonstrated by orthogonal methods with different measurement principles), it will not be possible to show comparability with quality data alone.

In order to address these specific challenges for RM bioprocess design, the standardisation community has started to address:

- Standards for application of parametric release to RM products
- Standards for pre-qualification of starting materials for RM products
- Standards addressing the characterisation conundrum of comparability/critical quality attributes (CQAs)

Particular reference will be made to existing and planned UK measurement research initiatives and pre-standards (Publicly Available Specifications) and their relationship to current ISO standardisation initiatives

Standardization for Regenerative Medicine – Japan's Contribution

Yutaka Yanagita

- Learn how standards can serve as common language for stakeholders and contribute to regenerative medicine
- Learn how ISO/TC 276, especially WG 4, is working toward establishment of common language
- Learn how Japan is contributing to such international standardization

Quality and Operations Track Session 2 – Applying Change Within Controlled Systems and Processes

Thursday, May 3, 15:45-17:00 ***Room 520BE***

Chair: Rosemarie Bell

As technologies evolve and new methods of improved manufacturing are developed, we need to be able to implement these new process changes into our clinical manufacturing to deliver the best possible outcome for patients and not be confined to the processes that were deployed in early phase development if they become outdated. Leading on from the what and the why, this session will discuss the lifecycle of a change by addressing the theory behind change managements through to a moderate change to a new cell expansion platform during a phase III clinical trial, and the wide range of impacts you need to consider and how you manage implementation to maintain a state of control.

Defining the Change

Steven Keizer

- Describe the overall process of Change Control.
- Demonstrate the functional purpose of the specific quality system.
- Increase awareness of the incorporation of the change control system into the broader continuous improvement of a quality system.

Regulatory Expectations on Managing Change during R&D through Clinical Product Manufacturing

Bangon Longsomboon

- Regulatory requirements and expectation from R&D thru Clinical Product Manufacturing
- How to manage change in manufacturing process to ensure compliance
- Provide examples on change implementation models.

Management of a Cell Expansion Platform Change During Clinical Trial Manufacturing

Gerry McKiernan

- Why you need to manage change
- How to plan effectively for a major change
- How one change can impact on several systems

Quality and Operations Track Session 3 – Regulation of MTMMS and ATMPS: Manufacturing, Non-Clinical, and Clinical Perspectives

Friday, May 4, 10:45-12:15 ***Room 520BE***

Chair: Shirley Bartido

Session aims to describe the regulated elements required by authorities in the preclinical assessment and manufacture of a reproducible, safe and efficacious product for its intended clinical use and method of clinical delivery.

GMP Manufacturing Considerations for Advanced Therapeutic Cellular Products

Shirley Bartido

- To provide an overall view of the different components that are regulated in the GMP manufacture of these advanced cellular therapeutics
- To highlight the critical parameters in the Quality assurance and control of advanced cellular therapeutics
- To provide a list of guidances from both US and EU regulatory authorities and highlight inherent differences in the regulation of manufacture of these advanced cellular therapeutics

Non-clinical Considerations in the Development of Cell and Gene Therapies

Julianne Smith

- Overview of nonclinical studies for early phase cell and gene therapy clinical trials
- Specific considerations for gene-edited therapies
- Comparison of EU and US nonclinical perspectives

Regulatory Challenges and Opportunities for Clinical Development of Cell and Gene Therapies

Elena Spanjaard

- Regulatory requirements for gene therapy clinical protocols in the US, EU and Japan
- Role for clinical trial site staff and institutional oversight bodies
- Available Regulatory pathways for cell and gene therapies

Quality and Operations Track Session 4 – Day to Day Operations of a Stem Cell Lab

Friday, May 4, 15:30-17:00 ***Room 520BE***

Chair: Heather Garrity

This session is focused mainly on day to day operations of a cell therapy program. It aims to highlight strategies proven effective in managing a high volume lab in addition to accommodating new trials as well as introduce challenges faced when balancing standard of care procedures, including HSCT, with the commercialization of CAR T and other novel cell therapies. The goal of this session is to give insight to members on how to develop a framework that encourages growth in a cell therapy lab while maintaining daily operations.

Making Novel Normal: Incorporating Commercial CAR T into the Daily Operations of a Lab

Karl Stasko

- Identify opportunities where a lab can impact strategies for commercial CAR T implementation in a transplant program.
- Learn how to incorporate commercial CAR T products into your current workflow.
- Discover how to adapt and respond to the challenges posed when "novel" becomes standard of care.

Joseph Schwartz

Implementing CAR-T Program

Christine Rosati

- Establish an Immune Effector Cells Therapy (IECT) Program within an existing pediatric oncology clinic
- Provide methods of creating a shared care model that works for all
- Develop strategies to ensure long term data capture and patient follow-up

Quality and Operations Track Session 5 – Data Integrity - Requirements, Opportunities, Management, Reconciliation

Saturday, May 5, 08:00-09:00 ***Room 520BE***

Chair: Gabrielle O'Sullivan

Data integrity is integral to every part of the cell therapy manufacturing and supply chain. All regulatory authorities focus on data integrity issues during cGMP inspections and data integrity problems are increasingly the basis for Warning Letters issued by the FDA. There is also burgeoning interest in developing flexible systems for managing data in multi-centre cell therapy supply cycles that are compliant and validated. Additionally, data integrity and clinical trials are affected by changes to data protection requirements and planning is essential to avoid risks in these areas. This session brings together experts that cover all of these areas. It will be useful to those involved in cell therapy manufacture, supply, clinical trials and regulation.

The aim of the session is to develop an understanding about what is required and what the possibilities are in various cell therapy manufacturing and supply settings.

The Future of Data Integrity for Cellular Therapies – Why You Need to Think About It Now

James Blackwell

- Impart the importance of data integrity for compliance, quality, patient safety, and operational excellence
- Provide an overview of data automation and integration's impact for future products
- Provide steps development organizations should take now

Using Software to Manage Multicentre Advanced Therapy Supply Cycles

Robert Margolin

The presentation will give an overview of considerations needed when introducing software systems to manage the supply cycle of advanced therapies at multiple sites. Project initiation, installation and validation of multi-centre software will be addressed to provide an insight into maintaining supply chain compliance using cloud-based software.

Data Integrity Challenges and Solutions in a Public Hospital Based Cell Therapy Manufacturing Facility

Zlatibor Velickovic

- Common data integrity issues in laboratories
- Risk assessment and management associated with data integrity
- Systems solutions and mechanisms for preventative planning

Reconciling Data Integrity Requirements and the GDPR

Alex Denoon

- Overview of requirements of GDPR
- A pragmatic attempt to reconcile the requirements for data integrity with the new European
- General Data Protection Regulation

Quality and Operations Track Session 6 – Challenges of cGMP Clean Room Facility for Cell Therapy Manufacturing in an Academic Setting

Saturday, May 5, 11:00-12:00 ***Room 520BE***

Chair: Nadim Mahmud

The challenges of clean room facility building can be wide, including allocation of appropriate budget and adequacy of future sustainability of operating cost. It requires adequate planning by a team of multidisciplinary stake holders. Building a clean room facility to an existing cell processing facility adds another layer of challenges, notably sustaining current services while preparing to switch to the new facility. The additional challenges include constraints on resources including personnel, equipment and operation schedules while ensuring quality of cellular products being manufactured during the transition period. Adequate planning will require identification of potential risks and measures to mitigate such risks during the relocation to the new facility taking into account about the quality of cellular products. Facility design, environmental monitoring (sterility) as well as validation of equipment and operation in the new facility should be carefully considered. Therefore, advanced planning having adequate resources including personnel and back up equipment to maintain operation simultaneously in both current and the new facility under validation will be beneficial. Taken together, a validation plan prepared well in advance including facility parameter verifications, relocation plan, etc. without disrupting ongoing services need to be developed and adhered to while room for flexibility in schedule will need to be accounted to avoid negative consequences.

Challenges to Build and Validate a cGMP Facility Without Interruption of Ongoing Graft Processing Services

Nadim Mahmud

- To gain insights about planning and validation of facility design for successful transition to a new cGMP laboratory without interrupting services.
- To gain knowledge about tiered approach of facility/ equipment and process validation for the new facility.
- To gain knowledge about ongoing validation of final cellular product during the transition and following completion of move to the new facility.

Risk Management for cGMP Academic Facility

Chy-Anh Tran

- Develop an understanding of risk management for cGMP compliant.
- Identify high-risk areas for cGMP academic facility.
- Obtain mitigation tools for risk management of cGMP academic facility.

How to Set up a GMP Compliant Clean Room for an Academic Vector Production

Aisha Khan

- Building and maintaining vector production clean room facilities
- Identifying critical steps in function of facility and in process
- Each user's understanding, participation and self-discipline

Quality and Operations Track Session 7 – Regulatory Expectations for the Manufacturing Control Strategy

Saturday, May 5, 15:15-16:30 ***Room 520BE***

Co-Chairs: Rosemarie Bell and Sowmya Viswanathan

CMC Challenges in Cell and Gene Therapies: a Health Canada Perspective

Christopher Storbeck

- To inform the audience of the role of the Biologics and Genetic Therapies Directorate (BGTD) in the evaluation of CMC data provided in support of Clinical Trial and marketing applications submitted to Health Canada.
- To inform the audience of Canadian regulatory approaches to some of the various CMC challenges specific to Cell and Gene Therapy products, with a focus on Biologic Potency Assays.
- To inform the audience of resources available to sponsors with respect to putting together a CMC package for Canada, including directly contacting the BGTD for consultation.

Introduction to Process Control Strategy

Karin Hoogendoorn

Get an understanding of what a pharmaceutical process control strategy is:

- What are the elements of biopharmaceutical process development?
- Target product profile and quality target product profile related to critical quality attributes, critical process parameters, and critical material attributes
- Two real life examples to explain the strategy applied:
- Monoclonal antibody
- Cell based medicinal product

Design and Optimisation of Process & Product Controls for Cell-based Products

Paula Salmikangas

- Understand the specificities of cells as pharmaceuticals
- Recognize the balance between process and product controls
- Give tools for optimal process design through examples

Strategies for Commercialization Track Sessions

Strategies for Commercialization Track Session 1 – Future Proofing Your Supply Chain; Using the Lessons of the Past to Create Commercially Viable Logistics Platforms for the Future

Thursday, May 3, 11:00-12:15 ***Room 518BC***

Chair: Simon Ellison

This session will use MACI, Strimvelis and Provenge as applied examples, then overlay the broader view of a CMO to identify lessons learned and suggest what supply chains of the future should look like.

Highlighting topics such as how to manage:

- Cost by reducing complexity
- Vein to vein journey as a single inter-related system
- Scale up/out by utilizing a logistics platform early in the development pathway

Lessons Learned from Currently Marketed Cell and Gene Therapies - Personal Reflections

Sven Kili

- This talk will provide an overview of Logistics used for marketed products and lessons learned
- What should be considered in setting up an effective Logistics platform

The Race Against Time – the Pioneering Supply Chain Behind the Delivery of PROVENGE, a Commercial Immunotherapy

Christina Yi

- Delivery of a time sensitive product requires a robust logistics network
- Proprietary systems are necessary to support the planning and delivery activities
- Third party transportation vendors are key to successful patient experiences

The Current and Future Landscape for Cell Therapy Deliverability: a CDMO Perspective

Robert Preti

Strategies for Commercialization Track Session 2 – How do Clinical Trial Design and Subsequent Learnings Impact Commercialization?

Thursday, May 3, 11:00-12:15 ***Room 520AD***

Chair: Uri Herzberg

As cell therapy is coming of age, it is evident that along the clinical opportunity, successful commercialization could be a major challenge. Hence, the design of clinical trials, in addition to including the endpoints required for registration, must address elements needed for commercialization. The session objectives will be to share the learnings and experiences of seasoned clinical leaders in the field and highlight the challenges that must still be addressed.

Specific objectives will include the following:

- Adequate Clinical design is key to getting to the appropriate patient profile for best response to the product. Specifically as reimbursement is easier for the patient that shows greater clinical benefit. Examples include patients sub-population in stroke, disease stage in diabetes, and disease severity in Crohn's
- Similarly, side effects, dosing and treatment expectations for physician and patients are important when formulating care givers' education around efficacy and safety / side effects, prior to commercialization
- Supply chain issues, formulations, efficiencies in every stage of the product life cycle training of health care providers as well as elements yet unknown (what we "do not know that we don't know")

Applying Lessons from Early Stage Clinical Trials to the Pivotal Phase III MASTERS-2 Ischemic Stroke Study

Robert W. Mays

- How to successfully translate pre-clinical observations with cellular therapies into clinical trial design and meaningful endpoints
- The importance of surrogate potency assay development
- The necessity for flexibility in product formulation and administration

Clinical Trial Data and Learnings Supporting Product Commercialization

Steven Fischkoff

- Data needed for payer packages
- Data needed for Formulary packages (Clinical's role is to collect operational data on how the below issues were dealt with during the trials and refine processes prior to launch)
- Data needed for Health Authorities such as NICE

Clinical Trial Design Incorporation of Commercial Considerations

Monica Luchi

- Utility of quality of life assessments
- Differentiation from competitor agents
- Biomarker development to assess likely responders

Strategies for Commercialization Track Session 3 – Tools and Automation Solutions for Accelerating Cell and Gene Therapy Development

Friday, May 4, 10:45-12:15 ***Room 518BC***

Chair: Julie Murrell

With recent approvals, the urgency for improvements in Cell and Gene Therapy Development are even more immediate than in the past. Here, we have perspectives from academia, biotech and suppliers:

- What are the drivers for change?
- What sort of technologies and innovations do we use to develop new products and reagents?
- How can we implement these tools into the development cycle?
- A panel discussion will follow the speaker presentations and will be open to audience questions.

Qasim Rafiq

Technologies for Radically Reducing Development Timelines of hMSC-based Therapeutic Products

Jon Rowley

Scale up of Allogeneic hMSC Manufacturing Processes can take several years and cost millions of dollars to develop and implement, and multiple automated technologies are required to achieve commercially relevant lot sizes. This presentation will outline lot size and technology requirements, the impact of supply chain on timelines, and present novel strategies that can be employed to radically shorten the time to implement these cutting edge manufacturing innovations.

Strategies and Methodologies for Selecting and Implementing New Technologies for Cell Therapy Processes

Gregory Russotti

- To describe the risk-based approach we followed in selecting new technologies for a cell therapy process
- To give examples of technologies that we implemented as well as some that we chose not to implement
- To demonstrate a data-driven methodology to assess whether the implementation of a new technology was worth the risk

Strategies for Commercialization Track Session 4 – 21st Century Global Regulatory Impact with Divergent Markets on Commercialization

Friday, May 4, 15:30-17:00 ***Room 518BC***

Chair: Julie Allickson

To provide listeners with a better understanding of the global regulatory environment and how different jurisdictions compare as described from companies commercializing their technology, we will focus on 3 areas of comparison and contrast for regulatory considerations in different countries/regions:

- Comparing and Contrasting US and JPN
- Comparing and Contrasting EU and JPN
- Australian Clinical Trials leading into JPN (and possible US/EU) clinical trials and/or strategic considerations on why Japan is an enticing location for clinical development of cellular therapies.

DiscGenics Overview. Regulatory Pathways in the US and Japan

Flagg Flanagan

- Familiarization with DGx Cell Therapy
- Experience with US regulatory pathway
- Experience with Japan regulatory pathway

21st Century Global Regulatory Impact with Divergent Markets on Commercialization

John Martin

- Japan's regenerative medicine regulatory environment has encouraged foreign regenerative medicine companies (like Regeneus) to pursue commercialisation in that market
- Japanese stakeholders have worked together effectively to create a positive environment for the accelerated development and commercialisation of RM technologies
- Japan's RM industry body, FIRM, has engaged globally (including in Australia) to promote the benefits of the government strategy and market opportunity

European ATMP regulation: Milestone or Hindrance?

Rainer Marksteiner

The European requirement for a GMP manufacturing authorization has created a common understanding of quality and release criteria of AMTPs between biotech companies and the competent authorities over the last 10 years.

The standard clinical development program (Phase I to III) is adjusted and approved for small molecules. Following this regulatory pathway with AMTPs could become a challenge because it does not address the unique characteristics of these products.

EMA offers a set of different pathways for the centralized marketing authorization in the EU. The challenge for each biotech company is to find the most suitable way for each ATMP. in special the "Hospital Exemption" became the subject of controversial debate.

Colin Novick
Panel Discussion: Julie Allicskon, Flagg Flanagan, John Martin, Rainer Marksteiner, Colin Novick, Michael Mendicino

Strategies for Commercialization Track Session 5 – Managing Patient Expectations and Reimbursement/Patient Advocacy/Market Access

Saturday, May 5, 8:00-9:00 ***Room 518BC***

Chair: Gerhard Bauer

This session will cover the patient and patient advocacy perspective in regards to reimbursement and market access for novel cellular therapies. Expectations and outcomes from these novel therapies for patients and providers will be examined and equitable access to such therapies for patients, particularly from different countries, will be discussed.

Making Cellular Therapy Products Available in the North American Market

Gerhard Bauer

Patient Access to CAR-T Before Approval

Dawn Driscoll

- Review the regulatory status of commercial car-t products (e.g. where are they available by Rx)
- Present options that patients have for accessing commercial car-t in countries where they are not yet approved.
- Present case studies from Australia, focusing on the impact to patients and their families, when a life-saving therapy is available in another country, but not one's own.

CAR-T Therapy, a Patient's Perspective

Douglas Olson

- Understand a patient's journey through a CAR T clinical trial
- Understand a patient's view on access to CAR T therapy
- Understand a patient's perspective on the financial issues of CAR T therapy

Strategies for Commercialization Track Session 6 – Growing the Pie: Has Investor Interest Increased for CGT post-approval of Kymriah, Yescarta and Luxturna?

Saturday, May 5, 11:00-12:00 ***Room 518BC***

Chair: Bill Milligan

How have the recent approvals of these two CD19 CAR T therapies and gene therapy influenced investor behaviour? in this session, we'll look at how investors perceive and value CGT investments versus other more traditional treatment modalities and explore what their biggest remaining concerns might be. in addition, we'll explore how to identify and engage the right investors and how to optimally position your company to raise capital. a must attend session in understanding what has changed for investors and how to leverage it going forward.

A Whale Swims into a Pond... Cell and Gene Therapy Hit the Market - Now What?

Patrick Rivers

- Articulating what investors care about for evaluating CGT companies
- Describing how investors characterize risk, and how recent CGT approvals impact the risk profile
- Theorizing the ripple effects that recent approvals have on capital deployment to CGT and biotech in general

Venture Capital Views on Investments in CGTs

Didier Leconte

- To share insights from venture capital firms, and provide guidelines on the topic

The European Perspective

Matthew Durdy

- Is Europe an alternative source of funds or a sub-sector of North America?
- How patient is Patient Capital?
- What are the trends and the opportunities?

Strategies for Commercialization Track Session 7 – ISCT-BPSA Joint Session: Next Generation Manufacturing for Cell and Gene Therapies

Saturday, May 5, 15:15-16:30 ***Room 518BC***

Co-Chairs: Ohad Karnieli and Eric Isberg

Defining Single-Use Systems for Cell & Gene Therapy Manufacturing

Dominic Clarke

- Describe the common single-use products and materials used for CGT manufacturing
- Discuss some of the key challenges of single-use systems
- Identify opportunities to optimize single-use materials for CGTs

Bioreactor Process Correlations and Sample-free Cell Expansion

Gary Pigeau

- Data and example-driven thought leadership on the future of cell therapy manufacturing.
- Development of understanding between continuous process data and sample based data, and the motivation for removal of the latter.
- Process definition, control charts and the path towards CGT manufacturing.

Supervisory Control Automation for Next Generation Cell Therapy Manufacturing

Randy Schweickart

- Next Generation cell therapy process equipment must accommodate data interfaces for integration into a Supervisory Control layer (S95 Level 2/3) to enable automated data transfers, PAT and unit operation integration, and higher level Production Control functions.
- A cellular therapy manufacturing facility Supervisory Control/Process Control System will have unique characteristics compared to typical implementations, most specifically the need to interface many embedded microprocessors and local data networks rather than PLCs connected to large arrays of I/O.
- One key differentiator between cell therapy manufacturing models will be the reconfigurability of a centralized manufacturing facility versus the deployability of a more rigidly integrated process platform.

Panel Discussion: Ohad Karnieli, Eric Isberg, Dominic Clarke, Gary Pigeau, Randy Schweickart, Shannon Eaker

Thank you to Our Industry Community Members

Patrons

BioLife Solutions — Biopreservation Tools for Cells, Tissues, and Organs

BlueRock Therapeutics

GE

Hitachi Chemical — Working On Wonders

MilliporeSigma

SALIAI

sartorius stedim biotech

ThermoFisher Scientific

Partners

Athersys

BD

Brooks Life Sciences

CCRM — Commercializing Living Therapies

CATAPULT

cell therapies

celularity

COOK REGENTEC

FRESENIUS KABI — caring for life

IrvineScientific

Lonza

MACS Miltenyi Biotec

TERUMOBCT

World Courier

zelluna IMMUNOTHERAPY

Supporters

AKRONBIOTECH

ASEPTIC TECHNOLOGIES

BI Biological Industries — Culture of Excellence

CellGenix

charterMedical

BioSharing

PALL Biotech

STEMINENT BIOTHERAPEUTICS

Contributors

Alliance of Advanced BioMedical Engineering — Powered by ASME

Beike Biotech

eppendorf

GADETA

Invetech

KeyBiologics

MaxCyte

Pluristem

psi PRESERVATION SOLUTIONS

SAINT-GOBAIN

scinogy

Conference Supporters

Platinum Sponsors

CellCAN
Regenerative Medicine and Cell Therapy Network

MACS Miltenyi Biotec

NOVARTIS

Gold Sponsor

KeyBiologics LLC

Silver Sponsors

BD

BioLife Solutions
BIOPRESERVATION TOOLS FOR CELLS, TISSUES, AND ORGANS

BI Biological Industries
Culture of Excellence

Burroughs Wellcome Fund

COOK REGENTEC

Fonds de recherche Santé Québec

GE

HMR Centre de recherche Hôpital Maisonneuve-Rosemont
Centre affilié à l'Université de Montréal

IrvineScientific

macopharma BIOTHERAPY
DESIGNED FOR LIFE

Roche

ThermoFisher SCIENTIFIC

Bronze Sponsors

BioLamina
REVOLUTIONIZING CELL CULTURE

Northern Therapeutics Inc.

Welcome Reception

Northern Therapeutics Inc.

Industry Networking Reception

CellCAN Regenerative Medicine and Cell Therapy Network

&

Canadian Pavilion Partners

Gala Entertainment Sponsor

DRAPER®

ISCT – CBA Cord Blood Series

ASBMT

Cord Blood Association

International Society ISCT Cell & Gene Therapy

Early Stage Professionals Networking Reception

MaxCyte®

WIFI

KeyBiologics, LLC

GRP Workshop

Burroughs Wellcome Fund

cell therapies

EMO

Educational Grant provided by cell therapies

MSC Workshop on Potency Assays

International Society ISCT Cell & Gene Therapy

RoosterBio®

ISCT Orthopedics Signature Series

International Society ISCT Cell & Gene Therapy

greyledge technologies

mtfbiologics

ORTHOFIX

medXcell

Osiris Therapeutics, Inc.

ANGIOCRINE BIOSCIENCE

ISCT-FACT Cell Therapy Quality Boot Camp

fact Foundation for the Accreditation of Cellular Therapy at the University of Nebraska Medical Center

International Society ISCT Cell & Gene Therapy

Canadian Cell & Gene Therapy Strategy Workshop

CellCAN Regenerative Medicine and Cell Therapy Network

International Society ISCT Cell & Gene Therapy

Corporate Session Summaries

Corporate Symposium

Wednesday May 2 | 16:30 – 18:30 | 520CF

MACS
Miltenyi Biotec

Emerging Technologies and Clinical Advancements in Cell Therapy

Chair: **Neehar Bhatia,** *PhD, Stanford School of Medicine, United States*
Speakers:

First clinical trials utilizing the automated CliniMACS Prodigy® TCT process
Claire Roddie, *PhD, UCL Cancer Institute, United Kingdom*

CAR T cell production at the Conconi Family Immunotherapy Lab using the CliniMACS Prodigy platform
John Webb, *PhD, British Columbia Cancer Agency, Canada*

Adoptive T cell therapy: Enabling technologies for clinical trials
Cassian Yee, *MD, The University of Texas MD Anderson Cancer Center, United States*

CAR T cells: progress and challenges
Crystal L Mackall, *MD, Stanford University, United States*

The Symposium will focus on clinical application and development of cell therapies.

Corporate Symposium

Thursday May 3 | 17:15 – 19:15 | Plenary Hall

NOVARTIS

What Makes CARs Work? – A Look Under the Hood

Chair: **Marcela Maus,** *MD, PhD, Massachusetts General Hospital, United States*
Speakers:

John Bell, *PhD, Ottawa Hospital Research Institute, Canada*

Abhijit Chakraborty, *PhD, Novartis Pharmaceuticals, United States*

Marcela Maus, *MD, PhD, Massachusetts General Hospital, United States*

Bijal Shah, *MD, Moffitt Cancer Center, United States*

This satellite symposium will serve as a forum in which the panelists will discuss key attributes of emerging chimeric antigen receptor (CAR) T-cell therapies, including:

- Contribution of various CAR domains to the efficacy and safety of CAR T-cell therapies
- Current understanding of CAR T-cell cellular kinetics in hematologic malignancies
- Role of lymphodepleting chemotherapy, mechanisms of resistance, and predictors of response, relapse, and safety

Corporate Tutorial

Thursday May 3 | 12:30 – 13:30 | 518BC

Improving Cell Therapy Production with Standardized and Consistent Flow Cytometry Assays and Sterile Fluorescence Activated Cell Sorting

Chair & Speaker: **Scott Bornheimer,** *PhD, BD Biosciences, United States*

Cells are frequently characterized during cell therapy production to track important subsets for research, process monitoring and QC for final release. Standardized flow cytometry assays that perform consistently across time, operators, instruments and sites are critical in this regard. BD will present technology and methods to meet these needs, based on IVD systems adapted for use in self-validated GMP cell processing. Consistency in cell starting materials, including isolation of target cells and removal of non-beneficial cells, is also important for consistent cell therapy production. Fluorescence activated cell sorting (FACS) can isolate cell subsets based on quantitative expression of multiple markers with excellent purity, viability and yield. We will highlight the current use of FACS in GMP cell processing applications and present technologies for sterile, closed and easy-to-use FACS to facilitate future use.

Corporate Tutorial

Friday May 4 | 12:30 – 13:30 | 518BC

The First Clinical Grade Virally Inactivated Human Platelet Lysate, MultiPL'i: A Powerful, Safe and Standardized Alternative to FBS to Expand Cell Therapy Products.

Chair: **Bruno Delorme,** *PhD, Macopharma, France*
Speakers:
Bruno Delorme, *PhD, Macopharma, France*
Andrew Finnerty, *Centre for Cell Manufacturing Ireland, Ireland*
Sabrina Viau, *PhD, Macopharma, France*
Jorge Burns, *PhD, University Politehnica of Bucharest, Romania*

Human platelet lysate (hPL) represents a powerful xeno-free alternative to fetal bovine serum (FBS) for human mesenchymal stem cell (hMSC) expansion. However, the characterization and the batch-to-batch standardization of such products still remain a challenge. In addition, the general chapter 5.2.12 of the European Pharmacopeia requires the addition of a step of viral inactivation during the production process of such raw material of biological origin used for cell-based and gene therapy medicinal products.

We will report the extensive characterization and document the robust standardization of our different clinical grade hPL products (MultiPL'), including growth factors (GF) contents, multiplex assay and biochemical and proteomic analysis. Data of comparison of hMSCs cultured with either FBS or MultiPL' will be presented (expansion, morphology, membrane marker expression, potential of differentiation and immunosuppressive properties). We will highlight some key characteristics of hMSC cultured in hPL. Importantly, we will also show that the use of standardized hPL improves the standardization of biological features of hMSCs. Finally, the efficacy of the gamma irradiation to inactivate a broad range of viruses in MultiPL' will be documented. The impact of the gamma irradiation on MultiPL' and the biological features of hMSCs cultured in MultiPL'i will be described.

Corporate Tutorial

Thursday May 3 | 07:30 – 08:30 | 518BC

Learning by Doing: Learn from Clinical and Manufacturing Leaders' Through Their Journey from Development to Delivery of Cellular Therapies

Speakers:

Allan Dietz, *PhD, Mayo Clinic, United States*

Marcela Maus, *MD, PhD, Massachusetts General Hospital, United States*

Bruce Levine, *PhD, University of Pennsylvania, United States*

Academic clinical institutions are fueling the remarkable advances in research, development and commercialization of cellular therapies. This progress is driving the need to adopt cGMP production standards during early development phases. Alongside industry, academic institutions must grapple with ways to assure product consistency, safety, and availability of the product. In this session panelists will discuss the evolution and challenges faced by clinical centers in their pursuit of developing and delivering new cellular therapies to the patients they serve.

Corporate Tutorial

Thursday May 3 | 07:30 – 08:30 | 519AB

Rapid Microbiological Methods in Meeting Unique QC and Product-Specific Validation Needs of Advanced Therapy Manufacturing

Chair:

Evonne R. Fearnot, *MSBME, Roche CustomBiotech, United States*

Speakers:

Alexey Bersenev, *MD, PhD, Yale University, United States*

Sowmya Viswanathan, *PhD, University Health Network, University of Toronto, Canada*

Rapid microbiological methods (RMMs) are essential for state-of-the-art manufacturing efficiency of advanced therapies because traditional testing methods do not provide shorter testing timelines and lower sample volume utilization. Some advanced therapies involve a manufacturing process and release timeline of just a few days making third-party testing impractical. There are ways to incorporate RMMs for QC testing, such as using a nucleic acid amplification technique as an alternative mycoplasma testing system after appropriate product-specific validation in-house. Leveraging available validation data facilitates a regulatory abbreviated qualification plan for RMMs. Several myths impede advanced therapy manufacturers from adopting these RMMs for QC testing. This session is intended to dispel these myths with accurate information and real-world experience.

Corporate Tutorial

Friday May 4 | 07:30 – 08:30 | 518BC

Training the Engineering Workforce for Cellular Therapy Manufacturing

Speaker:
Brian Behnke, *ASME Learning and Development, United States*

Leveraging the talents of skilled engineers working in the cell therapy space remains a challenge for many biopharmaceutical companies. Recognizing that foundational learning and standardization are critical success factors, the American Society of Mechanical Engineers (ASME) has developed a tool to begin filling this need: an introductory cellular therapy manufacturing eLearning course for engineers. Preview this solution that you can use to help educate your current engineering team or onboard your future workforce. Using real world examples and case studies, the course will cover end-to-end cell therapy manufacturing, emphasizing the engineering problems and processes and the critical roles engineers play.

Product Theatre Presentation

Thursday May 3 | 10:30 – 10:45 | Global Product Showcase

Simplify Upstream and Downstream Cell Therapy Processing with GE's Sefia™ S-2000 System.

Speaker:
Bertrand Foucaut, *GE Healthcare – Cell Therapy, Switzerland*

Cell processing takes place in a complex environment that poses many challenges. GE Healthcare Life Sciences provides solutions that simplify your everyday operations while maintaining flexibility and performance.

Our newest solution allows you to combine multiple workflow steps. The Sefia S-2000 system is suitable for both upstream and downstream processing. Use the same instrument for both – just select the appropriate combination of protocol software and disposable kits. With one software and kit combination you can prepare apheresis products. Set up the software to run volume reduction, platelet removal, and density gradient-based separation alone or automatically in sequence. With another software and kit combination you can perform downstream operations. Customize the sequence of harvesting, washing, and final formulation operations to fit your workflow needs.

Product Theatre Presentation

Thursday May 3 | 10:45 – 11:00 | Global Product Showcase

Expansion of Human Induced Pluripotent Stem Cells in Suspension Culture

Speaker:

Aletta Schnitzler, ***PhD,*** *MilliporeSigma, United States*

The therapeutic potential of human induced pluripotent stem cells (hiPSCs) is explored in a large array of indications, ranging from acute myocardial infarction to diabetes. The inefficiencies in some current differentiation protocols combined with the large numbers of cells recommended for clinical scale tissue engineering warrant the use of systems that are capable of generating large batches of hiPSCs in a controlled manner. In this study, a stirred-tank 3L bioreactor was used for the final expansion of hiPSCs as aggregates in a system providing pH, dissolved oxygen (DO), temperature and agitation control. Overall, a 125-fold expansion was achieved after a 14 day two-step process. The bioreactor-expanded hiPSCs retained expression of the pluripotency markers and formed tissues of each of the three germ layers. Moreover, hiPSCs cultured as aggregates were also directly differentiated to therapeutically-relevant cell types including cardiomyocytes. The results demonstrate the potential of hiPSC production in controlled stirred suspension systems that can support the production of large batches of cells for research and clinical applications.

Product Theatre Presentation

Thursday May 3 | 12:30 – 12:45 | Global Product Showcase

Get Lean: Streamlining Single Cell Analysis Workflow

Speaker:

Marilyne Levadoux-Martin, ***PhD,*** *Beckman Coulter Life Science, Canada*

While Flow Cytometry has been a core technology for advancing scientific research in cellular therapy, constant innovation is necessary to eliminate inefficient workflows, sources of variability and user introduced errors. Beckman Coulter's disruptive technologies are uniquely positioned to reshape your workflow without compromising data quality or compliance.

With Beckman Coulter's Complete Workflow Solution you can have:

- Consistent assay results by using our proven dried down antibody panels with **DURAClone Reagents**
- Outstanding sensitivity and electronic record management with the **CytoFLEX Flow Cytometer Platform**
- Traceable analysis and reporting through user management and LIS integration with **Kaluza Analysis Software**
- Automated staining protocol, from sample preparation through analysis, by directly integrating a **CytoFLEX** analyzer with a **Biomek i-Series Liquid Handler**

Product Theatre Presentation

Thursday May 3 | 12:45 – 13:00 | Global Product Showcase

CCRM Commercializing Living Therapies

Developing Closed and Integrated Processes for Cell Therapy Manufacturing

Speaker:
Aaron Dulgar-Tulloch, *PhD, BridGE, GE Healthcare, Canada*

As cell and gene therapies advance towards market approvals, there is a need for robust manufacturing solutions that are suitable for commercial scale production. Many cell manufacturing processes are currently being carried out with manual handling steps in an open manner, putting them at high risk for process variability. Our team is developing processes suitable for commercial scale manufacturing for both scale-up and scale-out cell production. As an example, we have demonstrated closure and integration of a T-cell production process by: automating the processing of input fresh and frozen apheresis units; closing both small- and large-scale T-cell culture using gas permeable bags and the Xuri™ W25 Cell Expansion System; and integrating cell culture and downstream harvest, wash, and formulation using the Sefia™ Cell Processing System. We have demonstrated a similar conversion from manual tissue culture to a closed and integrated bioreactor-based manufacturing of scaled-up pluripotent stem cells in a 10 L stirred tank reactor. With these strategies we are providing cell manufacturing solutions that can be readily adapted to a variety of customer-specific processes.

Product Theatre Presentation

Thursday May 3 | 13:00 – 13:15 | Global Product Showcase

MACS Miltenyi Biotec

Automated Clinical-Scale of Gene-Engineered T Cells Using the CliniMACS Prodigy

Speaker:
Michael Papadimitrious, *Miltenyi Biotec, Germany*

For over 25 years, Miltenyi Biotec has been providing technologies spanning the entire workflow of cell therapy, with solutions applicable from basic research to GMP manufacturing. The CliniMACS Prodigy is a cell manufacturing platform – one instrument that automates the handling steps in a single-use disposable tubing set. The example of a chimeric antigen receptor (CAR) T cell manufacturing process will be presented, demonstrating an integrated solution for cell selection, activation, transduction, and expansion.

Product Theatre Presentation

Thursday May 3 | 13:15 – 13:30 | Global Product Showcase

BE THE MATCH BioTherapies®

Auditing, Onboarding, and Managing Apheresis and Marrow Collection Networks to Support Late-Stage Clinical Study and Commercial Launch of Cell and Gene Therapies

Speaker:
Chris McClain, *Be The Match BioTherapies, United States*

One of the major obstacles faced by developers of emerging cell and gene therapies is providing reliable, nationwide access to apheresis and marrow collection centers. Over the course of its 30 year operating history, the National Marrow Donor Program (NMDP)/Be The Match has built and managed a national network of more than 80 apheresis centers and 70 collection centers. Through that network, NMDP/Be The Match has managed more than 74,000 stem cell harvests. Be The Match BioTherapies, a subsidiary of NMDP/Be The Match, will describe solutions that it provides to developers of cell and gene therapies to support late-stage clinical development and commercialization of therapies that rely on stem cell harvest for therapeutic starting material.

Product Theatre Presentation

Thursday May 3 | 15:15 – 15:45 | Global Product Showcase

IrvineScientific™

Chemically-Defined Culture Media for Advancing Cell-Based Immunotherapy Technology

Speaker:
Jessie Ni, *PhD, Irvine Scientific, United States*

Cell-based immunotherapy applications are widely captivating today due to their clinical potential to become life-saving therapies for cancer patients. Generation of sufficient, desired cell populations is an essential task for the successful development of cell-based immunotherapies, which require an effective, scalable, and consistent ex vivo process. A suitable chemically-defined (CD), animal-component-free (ACF) cells basal media for major immune cells, such as T-cells and natural killer (NK) cells, would significantly foster the establishment of such a process. By applying spent media analysis and the quality by design (QbD) approach, we examined the effects of various key media compositions such as amino acids, vitamins, minerals, and lipids on activated human peripheral blood-derived T-cell or NK cell expansion. The results from our studies were used to develop CD, ACF basal expansion media for desired T-cell and NK cell populations that are comparable to media containing serum, and further indicate the need to develop cells- and application-specific basal media to establish an optimal production process for a desired/targeted immune cell-based therapy under CD conditions.

Product Theatre Presentation

Friday May 4 | 10:15 – 10:45 | Global Product Showcase

BioLamina
REVOLUTIONIZING CELL CULTURE

CTG BioLaminin™ 521 – a Biologically Relevant Culture Matrix, Enabling Pre-Clinical Research Protocols to be Translated and Used for Clinical Trials.

Speaker:
Therese Kallur, ***PhD,*** *BioLamina, Sweden*

As an extension of our portfolio of hr laminin cell culture substrates, we now offer a cell therapy grade (CTG) laminin-521 matrix (BioLaminin™ 521) for use in research and manufacturing of Cell, Gene, or Tissue-Based Products.

CTG BioLaminin 521 is xeno-free and provides a defined surface for feeder-free culture of human pluripotent stem cells (ESC and iPSC), MSCs, most anchorage-dependent progenitors and differentiated cells such as RPE, hepatocytes, cardiomyocytes and neurons. CTG BioLaminin 521 recreates a biologically relevant milieu *in vitro*, promoting high survival and robust single-cell or colony expansion of human pluripotent stem cells, and subsequent cell lineage specification. The cells grow with maintained pluripotency, genetic integrity in a homogeneous monolayer, easy to monitor and maintain.

The substrate is robust, flexible and compliant with any culture medium and protocol. It allows an operator-independent culture maintenance and reliable, standardized protocols which can easily be adapted to automation platforms.

CTG BioLaminin 521 is produced according to FDA and EMEA guidelines (USP <1043>) and is aimed to be an ancillary material in the manufacturing cell products for therapeutic use. It is designed to aid users in the qualification process of raw material.

Product Theatre Presentation

Friday May 4 | 12:30 – 13:00 | Global Product Showcase

COOK® REGENTEC

Different Makes The Difference: The CellSeal® System

Speaker:
Sean Werner, ***PhD,*** *Cook Regentec, United States*

The CellSeal® System aims to fill the industry's need for a cryogenic storage and recovery process. For storing, it is a solution for leakage, breakage, and contamination in cryogenic storage. For packaging, it is a solution for consistent small-batch sealing and automated large-batch filling and sealing. For thawing, it is a solution for standardizing the thawing process through customizable profiles. Through these solutions, we will demonstrate how the CellSeal platform leads to significant gains in processing scale-up, quality, and standardization for cell and gene therapies.

Product Theatre Presentation

Friday May 4 | 13:00 – 13:15 | Global Product Showcase

CellCAN
Regenerative Medicine and Cell Therapy Network

More than 4800 Days of a Wonderful Second Life! William Brock's Inspiring Story!

Speakers:

William Brock, *Davies Ward Phillips & Vineberg LLP and CellCAN Board of Directors, Canada*

Craig Hasilo, *PhD Candidate, CellCAN, Canada*

Diagnosed in September 2004 with acute myelogenous leukemia, the Montreal lawyer William Brock thought he had just received a death sentence with very little time left to live. Within his own city of Montreal, he discovered a true gem in advanced healthcare: a centre where stem cell transplantation and cell therapy were provided at the highest standards. Shocked to learn that such cutting edge technology existed in Canada, Mr. Brock sought a referral and immediate treatment that would forever change his life and that of everyone around him.

Product Theatre Presentation

Friday May 4 | 13:15 – 13:30 | Global Product Showcase

CellCAN
Regenerative Medicine and Cell Therapy Network

How Well do You Know Canada's Cell & Gene Manufacturing Landscape?

Speaker:

Craig Hasilo, *PhD Candidate, CellCAN, Canada*

Canada is known for its excellence in research, outstanding publication record on cell & gene therapies and our collaborative culture. Our thriving RMCT ecosystem is on the brink of a major expansion. At the core of our strengths lie our expertise in cell & gene therapy manufacturing for early and later-phase multicenter clinical trials or commercialization efforts. But how well do you know what Canada has to offer in terms of manufacturing capacity? To what extent do Canadian cell & gene therapy manufacturing centres collaborate?

In this interactive session you will learn more about CellCAN's cell & gene manufacturing facilities and transversal cores, and how they share best practices through a collaborative online platform to improve the quality, safety and feasibility of cell & gene therapies. This unique collaboration will ultimately increase the capacity to rapidly and effectively migrate innovative treatment concepts into standard clinical practice.

Product Theatre Presentation

Friday May 4 | 15:15 – 15:30 | Global Product Showcase

AventaCell BioMedical Corp

What's New with Human Platelet Lysates?

Speaker:
William Milligan, *AventaCell BioMedical Corp., Ltd., Canada, United States, Taipei*

Human platelet lysates are now broadly used to replace FBS and early serum-free supplements for the isolation, expansion and production of multiple cells including; AD MSC, UC MSC, BM MSC, CIK, NK, dendritic cells, and primary tissue cell lines. AventaCell's UltraGROTM line offers hPL and fibrinogen-depleted hPL (FD hPL) options in both RUO and GMP grade supplements. UltraGROTM GMP products are used in cell therapy research and clinical development. Pathogen reduction will soon be available in GMP UltraGROTM products to better meet regulatory requirements and expand clinical development applications. UltraGROTM supplements have also been shown to effectively replace FBS and serum-free supplements for MSC expansion in bioreactors including; micro-carrier spinner flask bioreactors and the Quantum bioreactor (2016 & 2017 publications). We're also introducing UltraKURE – NK this year, our first hPL-based kit for NK cell expansion. For more product information, samples, and new developments, please come visit us in Booth #320.

Corporate Master Class

Friday May 4 | 17:15 – 17:45 | Global Product Showcase

ThermoFisher SCIENTIFIC

Identifying a Lentiviral Production Platform to Achieve Cost-Effective Scalable Manufacturing

Speaker:
Calley Hirsch, *CCRM, Canada*

Lentiviral vectors are gaining increasing momentum as a preferred genetic delivery tool for cell and gene therapies. Manufacturing lentivirus for advanced therapeutics has long centered around transient transfection of adherent cultures for vector production, however, these methods invariably suffer from scale-up, labor, cost and consistency challenges. As an approach to circumvent these problems, transient transfection of suspension cells has been proposed. In this presentation, a lentiviral production screen will be discussed where two GMP accessible suspension cell lines were paired with different media and transfection reagents to identify a lentiviral vector platform that consistently yields the highest titer and lowest production cost. Based on these finding, internal scale-up efforts are ongoing to develop a readily adaptable cGMP lentiviral manufacturing process.

TMC PHARMA

The Global Pharmaceutical Development Organisation

Greater than 10 years working with stem cell and advanced therapy products - pioneered the first EU study in human embryonic stem cells

A unique service provider offering focussed solutions with full life-cycle expertise

Support 'from concept to patient'

True experts working in partnership with clients

Come and meet us @ Booth 219

- Non Clinical
- Medical
- Clinical
- PV
- Regulatory
- Post Marketing

visit **www.tmcpharma.com** contact **info@TMCPharma.com**

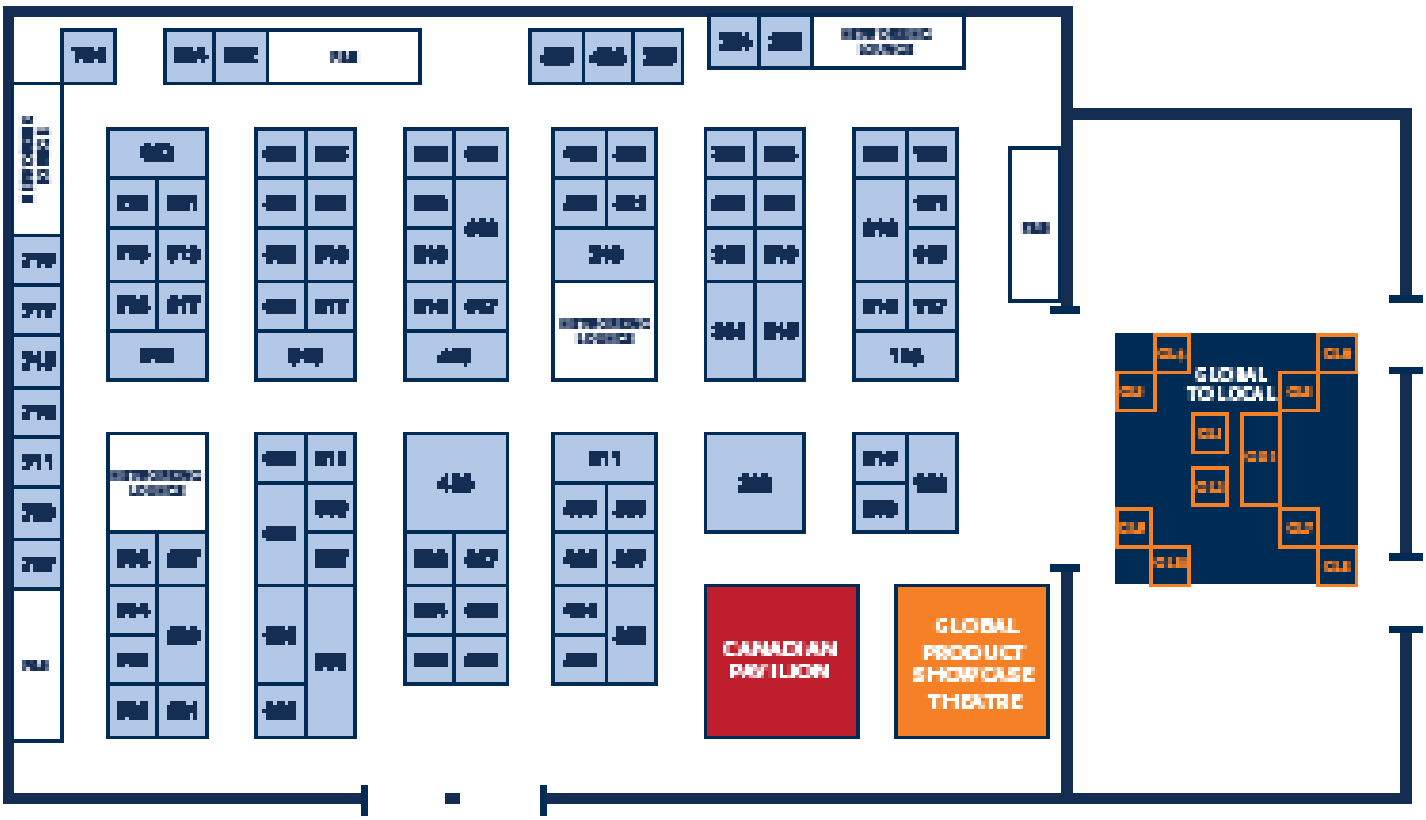
Tools to...

Cook Regentec creates tools to help you build your therapies from concept to commercialization.

COOK® REGENTEC

Stop by booth #501 to learn more.

Exhibit Hall Floorplan



Exhibitor Listing

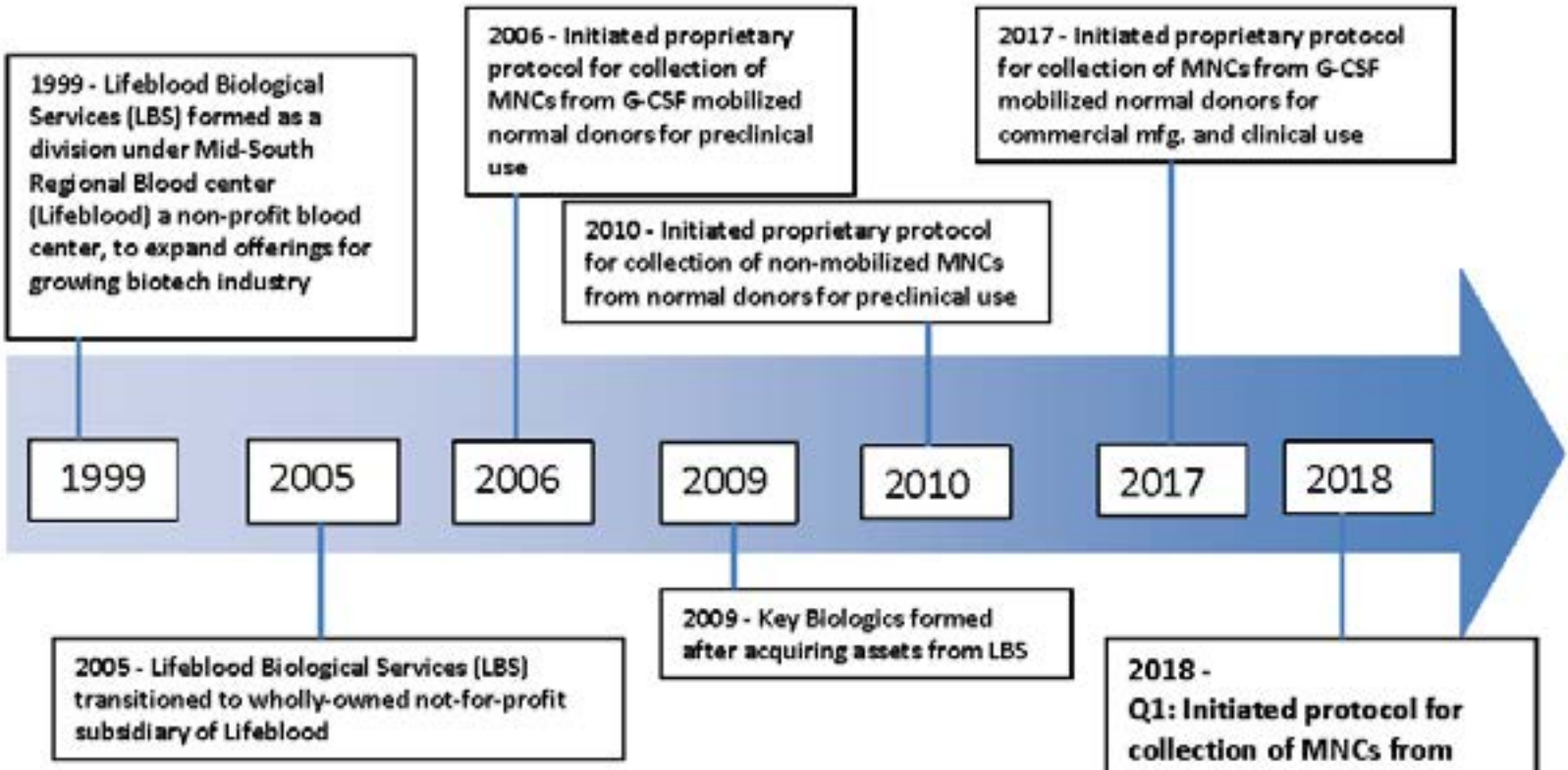
Exhibitor Company	Booth Number(s)
Akron Biotech	700
AllCells	502
AMSBIO	406
Aseptic Technologies	117
AventaCell BioMedical Corp. Ltd. / PELOBiotech GmbH	320
BC Regenerative Medicine Network	Canada Pavilion
BD Biosciences	314
Be The Match BioTherapies	518
Beckman Coulter Life Sciences	719
Bell Biosystems, Inc.	621
BioCanRx	Canada Pavilion
BioLamina	216
BioLife Solutions	209
BI Biological Industries	409
Bionique Testing Laboratories, Inc.	417
Bioquell	623
BioSpherix Medical	215
Bio-Techne	720
BloodCenter of Wisconsin	716
Brooks Life Science Systems	607
C3i	Canada Pavilion
CEFO	511
Cell Therapies Pty Ltd	600
CellCAN	Canada Pavilion
Cellex Cell Professionals	123
CellGenix	506
Centre for Commercialization of Regenerative Medicine (CCRM)	Canada Pavilion
Charter Medical	210
ChemoMetec	223
COMECER GROUP	707
Compass Biomedical	521
Cook Regentec	501
Cord Blood Association	704
Corning Life Sciences	606
Cryo Bio System	702
Custom BioGenic Systems	602
Digi-Trax	402
Draper	618
Eceptionist	610

Exhibitor Company	Booth Number(s)
Entegris	423
Eppendorf	425
Esco VacciXcell	218
Extract Technology	307
FACT	706
FloDesign Sonics	619
Fresenius Kabi	419
GE Healthcare	515
GenCure	715
GenesisBPS	321
GOLD SIM International Group	724
HemaCare Corporation	208
Héma-Québec	Canada Pavilion
ICCBBA	325
Immudex	601
Instant Systems	323
Irvine Scientific	303
Japanese Society for Regenerative Medicine (JSRM)	324
KBI Biopharma	709
Key Biologics	403
Macopharma	615
Mak-System	519
MaxCyte	309
McGill Stem Cell and Regenerative Medicine Network	Canada Pavilion
Mediware	507
Mill Creek Life Sciences	523
MilliporeSigma	222
Miltenyi Biotec	415
MVE	504
National Research Council of Canada	Canada Pavilion
Nordmark Biochemicals	119
Novartis Oncology	115
Ontario Institute for Regenerative Medicine	Canada Pavilion
OriGen Biomedical	713
PACT	318
Pall Life Sciences	109
PBS Biotech	405
PeproTech	420
RegMedNet	221
RENOLIT Medical	522

Exhibitor Company	Booth Number(s)
Réseau Thécell Network	Canada Pavilion
Roche Diagnostics	624
ROKIT	717
RoosterBio	225
Saint-Gobain	603
Sartorius Stedim Biotech	616
Savsu Technologies	718
Scinogy	404
Scinus Cell Expansion BV	711
Stem Cell Network	Canada Pavilion
STEM CELLS Journals	520
StemBioSys, Inc	620
stemTrak	516
Synex Consulting Ltd	509
Technoflex	408
Terumo BCT	311
Thermo Fisher Scientific	319
ThermoGenesis	322
time:matters	622
Title21 Health Solutions	422
TMC Pharma Services	219
TrakCel	407
Waisman Biomanufacturing	121
West Pharmaceutical Services	517
Wilson Wolf Corporation	617
World Courier	424

Global to Local	Booth Number(s)
The Baylor College Center for Cell and Gene Therapy	GL2
Children's National Health System	GL10
Clinical Cell and Vaccine Production Facility (CVPF)/ University of Pennsylvania	GL9
Comprehensive Cell Solutions	GL8
Cytotherapy, ISCT Official Journal	G11
Dana-Farber Cancer Institute	GL7
Fred Hutchinson Cancer Research Center	GL4
Miami School of Medicine	GL5
Stanford Laboratory for Cell and Gene Medicine	GL3
The University of Wisconsin Program for Advanced Cellular Therapy	GL1
Tulane University	GL6

KeyBiologics LLC



With nearly 20 years of experience in support of the cell therapy industry, we are the preferred partner for sourcing cells and related services for all the right reasons:

- Pricing
- Quality
- Customer Service
- Knowledge

Visit us at booth 403 to learn more about how we can help you succeed.

www.keybiologics.com

info@keybiologics.com

AT-CLOSED VIAL® TECHNOLOGY

Discover the industry leading technology for aseptic fill&finish of autologous and allogeneic products, based on a closed vial ensuring 100% of Container Closure Integrity during cryostorage.

For more details meet us at BOOTH 117

ASEPTIC TECHNOLOGIES

Exhibitor and Supporter Directory

Akron Biotech — Booth 700

Akron Biotech is a global supplier that develops, manufactures, and markets ancillary materials including cytokines, cryomedias, and human-derived products under cGMP compliance and offers a range of services including bioassay development, fill & finish, and custom packaging for the development and commercialization of cell and gene therapies and engineered tissues, serving our customers around the world from development to bedside.

AllCells LLC — Booth 502

Founded in 1998, AllCells is a biotechnology company dedicated to providing global researchers and biomanufacturing organizations with a dependable supply of biologically relevant, high quality primary cells that enable drug discovery, development, and manufacturing of cell therapies. Leveraging its expertise in hematology and immunology cell isolations, combined with on-site whole blood and bone marrow clinical collection operations, AllCells maintains direct access to a large repository of diverse donor profiles that can be further screened and qualified for biomedical applications. Utilizing its contiguous cell processing facilities, freshly collected tissue is immediately processed into purified cell types with unparalleled quality consistency. This optimal work-flow provides for the highest viability, purity, and cell count available. Fresh or frozen cells are shipped worldwide, or fresh cells can be immediately utilized in fee-for-service cell-based assays ("Bioservices") designed according to customer criteria – delivering cell culture results/data to meet client project timelines. For more information visit, www.allcells.com.

Alliance of Advanced Biomedical Engineering (AABME) — Not Exhibiting

The Alliance of Advanced Biomedical Engineering (AABME) is an ASME initiative designed to stimulate biomedical innovation by bringing together and providing resources to the biomedical engineering community.

AABME is for engineers, biologists, clinicians, scientists and researchers who have a passion for advancing human health. Our unique forum unites the key players in the bioengineering arena. Visit us at AABME.org.

AMSBIO LLC — Booth 406

AMSBIO is a long-established supplier of high-quality tools and technologies to support stem cell and cell therapy research. Our extensive product portfolio supports every facet of stem cell research ranging from stem cell sources, iPSC reprogramming agents, and ready-to-use feeder cells to advanced xeno-free GMP grade cryopreservation media.

Together with a comprehensive range of stem cell characterization products, differentiation reagents, and unique assay platforms, AMSBIO is proud to supply the industry's largest selection of recombinant extracellular matrices, and xeno-free culture medium offering superior high colony forming efficiency.

At the very heart of AMSBIO's core principles are quality and innovation, constantly seeking out new and innovative products to accelerate your research programme. We back our products with expert customer technical support; offering custom products to your specifications and can directly translate our in-depth knowledge to support your research programme through our contract research laboratories.

Many of our products are now available as GMP grade with appropriate Drug Master Files submitted. Visit www.amsbio.com or contact us info@amsbio.com

American Society for Blood and Marrow Transplantation (ASBMT) — Not Exhibiting

The American Society for Blood and Marrow Transplantation (ASBMT) is a professional society of more than 2,200 physicians, investigators and other health care professionals from over 45 countries. The Society is dedicated to advancing the science and clinical care for patients who require blood and marrow transplants for blood cancers and other deadly diseases.

Aseptic Technologies — Booth 117

ASEPTIC TECHNOLOGIES develops, manufactures and markets innovative aseptic solution for the BioPharmaceutical industry.

Its equipment and devices are designed to provide safer & easier operations for aseptic fill and finish, the best-known being the

AT-Closed Vial technology. The AT-Closed Vial is manufactured in ISO 5 clean room with the stopper secured in place and gamma sterilized. Vial size ranges from 1 to 50ml.

The filling of these ready-to-use vials is performed by a special needle piercing the stopper and dispensing the drug product. The stopper is then immediately resealed by a laser and a cap is snap-fitted on it.

Various equipment offer an easy scale-up from manual to fully automated operation.

The benefits of the technology are: better sterility assurance, reduced investments, increased safety during whole supply chain, strong operating cost reduction.

The AT-Closed Vial technology is widely used in the Cell and Gene Therapies as it offers uncompromised container closure integrity even during storage at cryogenic temperature.

www.aseptictech.com

AventaCell BioMedical Corp

AventaCell BioMedical Corp. Ltd. / PELOBiotech GmbH — Booth 320

AventaCell BioMedical Corp. Ltd. (Taipei, TW & Atlanta, GA, USA) is one of the leading global companies devoted to developing, manufacturing and supplying novel human platelet-derived products for use in cell culture, tissue regeneration applications and drug development. AventaCell offers human-derived solutions for use in translational research and development of cell and tissue-based therapies. AventaCell plans to launch gamma irradiated UltraGROTM products in 2018, to meet the demand of regulatory authorities for pathogen reduced hPL / FD hPl supplements to support end users advancing cell therapies to clinical development and commercialization. The Company is also launching UltraKURETM-NK, an hPL-based multi-component kit for NK cell culture, developed to meet the need for animal component-free NK cell expansion and production. Research has demonstrated the ability to effectively replace FBS with UltraGROTM supplements in various 3-D bioreactor systems (e.g. micro-carrier spinner flasks, Quantum System) for MSC production. The demand for safe, efficient, cost-effective cell expansion and production is rapidly increasing as a result of the tremendous growth in CGT and Regenmed products research and development. AventaCell is committed to supplying the industry with research grade, GMP grade, and gamma-irradiated animal serum-free products, which can accelerate the research, clinical development and commercialization of safe, effective and COG-efficient cell and tissue-based therapeutics. Please join us, and Pelobiotech (an EU distributor / partner), to arrange samples and for more information in Booth #320.

BCREGMED

BCRegMed — Canadian Pavilion

The British Columbia Regenerative Medicine Initiative (BCRegMed), is a funded Research Excellence Cluster whose mandate is to unite academic, industry and government entities to catalyze the rapidly expanding cellular and regenerative medicine community in BC. The group aims to establish BC as a premier destination for the discovery, development and implementation of cellular and regenerative medicine therapies and technologies. www.bcregmed.ca

BD

BD Biosciences — Booth 314

BD is a global medical technology company that is advancing the world of health by improving medical discovery, diagnostics and the delivery of care. BD leads in patient and healthcare worker safety and the technologies that enable medical research and clinical laboratories. The company provides innovative solutions that help advance medical research and genomics, enhance the diagnosis of infectious disease and cancer, improve medication management, promote infection prevention, equip surgical and interventional procedures and support the management of diabetes. For more information: www.bdbiosciences.com

BE THE MATCH BioTherapies®

Be The Match BioTherapies — Booth 518

Be The Match BioTherapies partners with organizations pursuing life-saving cellular therapies in every stage of development – from discovery through commercialization. Built on the foundation established over the last 30 years by the National Marrow Donor Program/Be The Match, we have unparalleled experience managing cellular therapies. Our cell therapy supply chain delivery for autologous or allogeneic therapies is enabled by high-touch, personalized case management and a robust, customizable technology platform, MatchSource. Our experience in cell sourcing and collection allows us to provide cells consented for research, clinical or commercial use. Researchers have access to clinical trial services through our research program, the CIBMTR. And, we have the infrastructure in place to collect, store and analyze patient samples post-cell or gene therapy treatment at the time points required by regulatory authorities. BeTheMatchBioTherapies.com

BECKMAN COULTER Life Sciences

Beckman Coulter Life Sciences — Booth 719

Beckman Coulter Life Sciences is dedicated to improving the health of people around the world. The company's global leadership and world-class service and support delivers sophisticated instrument systems, reagents and services to life science researchers in academic and commercial laboratories, enabling new discoveries in biology-based research and development.

Bell Biosystems, Inc. — Booth 621

Bell Biosystems, is a Bay Area based, emerging biotools company commercializing in vivo tracking and MRI contrast agent technologies for cell therapies. Our first product, the Magnelle® Cell Labeling and Tracking solution, provides precise location and viability data under MRI imaging for in vivo transplanted cells. Magnelle-powered(tm) pre-clinical studies are disrupting the way the regenerative medicine marketplace assesses cellular therapies by providing actionable insights for therapeutic durability including therapeutic cell viability feedback and localization of the transplanted cells.

BI Biological Industries — Booth 409

Biological Industries manufacturing expertise of cell culture products extends over 35 years. BI products include: The Nutristem® range of stem cell media which have become the gold standard in research and clinical applications, helping to advance stem cell based therapies.

In addition, BI supplies related products for stem cell culture and differentiation, including animal component-free freezing media, xeno-free attachment solution and animal component-free cell dissociation solutions.

Bio-Process Systems Alliance (BPSA) — Not Exhibiting

BPSA's Mission is to facilitate, globally, the development and manufacturing of biopharmaceuticals through the implementation of robust, safe and sustainable Single-Use Technologies.

BioCanRx — Canadian Pavilion

BioCanRx – Canada's Immunotherapy Network:

The BioCanRx network is accelerating to the clinic Canada's most promising and innovative cancer biotherapeutics designed to save lives and enable a better quality of life. BioCanRx invests in innovations and the best the field has to offer, always looking for a clear path to the clinic for the benefit of patients. BioCanRx works in partnership with industry, charities and other agencies to translate immune-based technologies from the lab into early phase clinical trials, and addresses socio-economic considerations necessary for their adoption by health-care systems. Through an innovative, collaborative funding process, BioCanRx is becoming a world-leader in the translation, manufacture and adoption of innovative cancer immunotherapies for the benefit of all patients. The network is developing and attracting the talent needed for a thriving health biotechnology sector in Canada. BioCanRx receives funding from the federal government's Networks of Centres of Excellence, and support from industry, the provinces and many national charities.

BioLamina AB — Booth 216

BioLamina offers a wide a variety of chemically defined and xeno-free hr laminin cell culture matrices for reliable expansion and differentiation of pluripotent cells and for maintenance of specialized cell types, such hepatocytes, cardiomyocytes and neural cells. Laminin-521 is also available as a cell therapy grade. The laminin cell culture matrices allow you to imitate the natural, cell-specific niche in vitro for improved cell functionality.

BioLife Solutions, Inc. — Booth 209

BioLife Solutions is the leading developer, manufacturer and supplier of proprietary clinical grade cell and tissue hypothermic storage and cryopreservation freeze media for cells and tissues. Our proprietary HypoThermosol® and CryoStor® platform of solutions are highly valued in the regenerative medicine, biobanking and drug discovery markets. Our biopreservation media products are serum-free and protein-free, fully defined, and are formulated to reduce preservation-induced cell damage and death; offering commercial companies and clinical researchers significant improvement in shelf life and post-preservation viability and function. For more information please visit www.biolifesolutions.com and follow BioLife on Twitter.

Bionique Testing Laboratories, Inc. — Booth 417

For over 25 years, Bionique Testing Laboratories has been a leading, worldwide provider of mycoplasma testing services for the pharmaceutical, biotech, and life science industries. We offer a wide array of testing methods from relevant compendia to a GMP compliant Real-Time PCR assay to support your needs from concept to commercialization for cellular therapies. Our vast experience extends to development of Rapid Microbial Methods and validation services. Our scientific expertise is backed by our commitment to quality and excellence in customer service.

Bioquell — Booth 623

Cell therapy companies are experiencing rapid, continuous and safer production with Bioquell decontamination solutions and the Bioquell Qube isolator. Integrated with Bioquell's Hydrogen Peroxide Vapour and capable of creating an ISO 5/Grade A space while hosted in a lower grade room, the Bioquell Qube is a modular isolator as innovative as your work. From its polypropylene construction and ease of installation to its integrated features and options, the Bioquell Qube is unlike traditional stainless steel isolators. Bioquell's proprietary 35% Hydrogen Peroxide Vapour leads the way in providing decontamination solutions with systems and services rapidly eliminating bioburden on every exposed surface, making Bioquell technology the ideal fit for creating an aseptic environment to perform time sensitive manipulations.

BIOSPHERIX MEDICAL — Booth 215

BioSpherix will be exhibiting the Xvivo System, the only cytocentric barrier isolator optimized for cells. Economical alternative to cleanrooms for cGMP compliant production of cells for clinical use. Stop by BioSpherix booth to learn more about the only total quality platform for cells!

Total quality recognizes that for best cell potency, cells need full-time optimization of all critical cell process parameters. Total quality recognizes that all typical negative side effects of machines on cells (particles, heat, vibration, etc.) must be neutralized and those machines must be protected from dust, aerosols, and corrosion. Total quality recognizes that each entire cell production line (all manual and automated steps) must be protected from microbial contamination. Total quality recognizes that all personnel must be fully protected from cells harboring virus, vectors, prion, and other pathogens. Total quality recognizes that scaling up and out must be efficient and cost efficient.

Bio-Techne — Booth 720

Bio-Techne empowers researchers in Life Sciences and Clinical Diagnostics by providing high-quality reagents, instruments, custom manufacturing, and testing services. Our family of brands creates a unique portfolio of products and services. Science is our passion; it drives us to collaborate, develop, and manufacture award-winning tools that help researchers achieve reproducible and consistent results. Whether you are at the cutting edge of academic research, translating basic discoveries to therapeutic leads, or at a facility that requires the highest level of diagnostic testing, our innovative products and services provide the solutions you need to achieve success.

BloodCenter of Wisconsin — Booth 716

The BloodCenter of Wisconsin is a world-renown organization with expertise in blood services, diagnostics and cell therapy support. With access to a very diverse range of donors with known HLA types through our Versiti network we can provide investigators and manufacturers with material and services key to making your research or your new drug successful. Our expertise in blood science, clinical diagnostics and medical consulting makes Versiti the perfect partner for drug discovery, preclinical development and cell therapy support. Our blood products include whole blood, apheresis collected platelets, human serum, human plasma and enriched PBMCs collected by apheresis (LeukoPaks) with or without G-CSF mobilization, all from HLA characterized donors. Versiti is also highly experienced in low to high resolution HLA typing as well as HLA antibody screening and identification. At our Michigan facility Versiti operates a Cord Blood program that can provide cord blood derived stem cells for transplant programs as well as a limited number of cord blood units for research.

Brooks Life Science Systems — Booth 607

Brooks Life Sciences, a division of Brooks Automation, Inc. (Nasdaq: BRKS), is the leading worldwide provider of innovative and comprehensive sample lifecycle management solutions for research and development organizations. Our solutions include automated storage systems, sample consumables and instruments, cell cryopreservation storage products, onsite and offsite temperature-controlled storage, global cold-chain logistics and relocation services, sample preparation and bioprocessing solutions, innovative informatics systems and technology services. Our team of sample management experts, deliver high quality, flexible sample management products, services and technology solutions that support hundreds of research-based organizations around the world, including the top 20 biopharmaceutical companies, top academic research organizations and government agencies. Our industry-leading sample management solutions enable our customers to unlock sample intelligence and advance scientific research in order to deliver healthier and brighter tomorrows to individuals around the world. www.brookslifesciences.com

C3i — Canadian Pavilion

"Canadian Expertise in Cancer Immunotherapy and Regenerative Medicine"

C3i provides an integrated structure to accelerate the discovery, development, commercialization and access to innovative cancer immunotherapies and regenerative medicine.

CEFO
CELL ENGINEERING FOR ORIGIN

CEFO — Booth 511

CEFO Co., Ltd. is a company specialized in stem cell research and development. We develop cell therapy products that are regarded as next-generation drugs in the pharmaceutical bio market, and culture elements including culture medium to create new value. In addition, we provide contract research service such as cell culture, in vitro efficacy and toxicity evaluation, and have a wide variety of primary cells and self-developed culture elements to develop advanced in vitro screening models and cell therapy products. The main development items are human cell therapy products for bone disorders and animal cell therapy products for companion or industrial animals. In accordance with the name of Cell Engineering For Origin, CEFO Co., Ltd. is establishing three-dimensional(3D) culture and differentiation system away from the 2D culture system. We lead the evolution of biotechnology by implementing Origin with these engineering techniques.

cell therapies

Cell Therapies Pty Ltd — Booth 600

Cell Therapies Pty Ltd (CTPL) is a leading cGMP manufacturer and distributor of cellular therapies. For over 15 years, CTPL has operated TGA regulated cell processing facilities from our home in Melbourne Australia, where we have strong clinical ties across multiple therapeutic areas. Our cutting-edge facilities and capabilities are commercially available on a fee-for-service basis and have been used by the world's leading cellular therapies companies. We have successfully helped develop products and processes that are compliant with global regulatory and cGMP requirements and have successfully integrated with global manufacturing networks.

We provide the essential process development, manufacturing and deployment infrastructure for the safe, reliable and scalable delivery of cellular therapies into clinical trials and for commercial therapeutic use globally. Our expertise is "needle-to-needle", ensuring complete product control from collection management (especially apheresis) to cell processing to distribution for clinical or commercial supply.

Cell Therapies has expanded its network with a strategic alliance with PharmaBio in Japan, offering a single gateway to Asia. We have also added an apheresis site management service line to provide control over this critical starting material. And now we are perfecting an exciting new nanoparticle tracking technology for imaging the distribution and persistence of therapeutic cells in vivo. Visit us at www.celltherapies.com.au or at our Booth 600.

CellCAN
Regenerative Medicine and Cell Therapy Network

CellCAN — Canadian Pavilion

CellCAN is a pan-Canadian non-profit organization established in 2014 that is part of the Government of Canada's Networks of Centers of Excellence. Our mission is to mobilize stakeholders and knowledge across Canada to significantly advance regenerative medicine and cell therapy research and clinical development. More specifically, our goal is to improve quality, safety and feasibility of cell and gene therapy in Canada through optimal manufacturing practices. CellCAN strives to fully exploit this potential by providing an unprecedented level of collaboration among all key players in the field of cell and gene therapy by bringing together Canada's leading cell manufacturing centers and transversal cores (manufacturing product characterization, bioengineering, ethical and legal regulatory policy) into a common seal of quality for the benefit of Canadian patients. For more information, visit www.cellcan.com.

cellex.
Cell Professionals

Cellex Cell Professionals — Booth 123

Cellex Cell Professionals is offering a broad spectrum of services for clinical as well as for research institutions in the field of cellular therapies. Our apheresis centers have all techniques for hematopoietic blood stem cells, lymphocytes, erythrocytes and granulocytes for patients and healthy donors for clinical use and research purposes. Furthermore we facilitate a database – CellCommunity - for lymphocyte donations which can be requested for research intentions. In our state-of-the-art GMP facility cellular subsets can be separated and frozen but also genetically modified and analyzed. But we take also care for rapid and safe transports – cryopreserved and on board - to worldwide destinations as well as the related paper work and the complete organizational procedure of cell therapy runs. Ask us if you would like to have other specialized services - we will always find a solution. Our great team of more than 200 employees has more than 15 years of experience and would be happy to serve you.

CellGenix

CellGenix GmbH — Booth 506

CellGenix is a leading global supplier of high quality reagents and tools in the expanding market of cell and gene therapy and regenerative medicine.

As the first company to obtain a GMP manufacturing authorization for cell processing in Europe, CellGenix has more than 20 years of expertise in the development and GMP manufacturing of cell therapy products. Our products are used worldwide in clinical trials by academia and industry partners.

To ensure a seamless transition from research to commercialization

we offer our customers a comprehensive product portfolio together with expert regulatory and technical support. Included in our product portfolio are cytokines, serum-free media and closed cell culture systems. Our products combine a maximum of quality and safety with excellent performance due to the state-of-the-art production, stringent in-house quality control and comprehensive documentation. All these factors help to simplify qualification and validation for your market authorization.

CellGenix operates a state-of-the-art GMP facility for production of recombinant proteins and cell processing in Freiburg, Germany. A subsidiary is located near Boston in Portsmouth, NH/USA.

For more information, visit www.cellgenix.com

CCRM Commercializing Living Therapies

Centre for Commercialization of Regenerative Medicine — Canadian Pavilion

CCRM, a Canadian not-for-profit organization funded by the Government of Canada, the Province of Ontario, and leading academic and industry partners, supports the development of regenerative medicines and associated enabling technologies, with a specific focus on cell and gene therapy. A network of academic researchers, leading companies, strategic investors and entrepreneurs, CCRM aims to accelerate the translation of scientific discovery into new companies and marketable products for patients, with specialized teams, funding and infrastructure. CCRM sources and evaluates intellectual property from around the globe, offers various consulting services, conducts development projects with partners, and establishes new companies built around strategic bundles of intellectual property. CCRM has a fully resourced development facility used to both evaluate and advance technologies, a Centre for Advanced Therapeutic Cell Technologies and a (coming soon!) GMP facility within a 40,000 square foot office designed for advanced cell manufacturing. CCRM is the commercialization partner of the Ontario Institute for Regenerative Medicine and the University of Toronto's Medicine by Design. CCRM is hosted by the University of Toronto and was launched in Toronto's Discovery District on June 14, 2011. Visit us at www.ccrm.ca

CharterMedical

Charter Medical, Ltd. — Booth 210

Charter Medical, Ltd. is a subsidiary of Fenner PLC (LSE: FENR). We have a 25 year history of developing and providing specialty single-use products to the blood transfusion, cell therapy, biotechnology and pharmaceutical markets. Our 15,000 square feet of clean room space located in Winston-Salem, NC, USA is ISO 13485 certified and FDA registered. Focus is on designing and supplying single-use solutions for cell growth, frozen storage and biological fluid handling. Charter Medical is committed to providing quality products and services with and experienced staff dedicated to exceeding customer expectations from product development, delivery and implementation.

chemometec

ChemoMetec — Booth 223

ChemoMetec is a Danish founded company, which specializes in development, manufacturing and sales of high quality automated Cell Counters, Advanced Cell Analyzers and Image Cytometers to help streamline research and production processes for maximum efficiency. ChemoMetec instruments are based on a patented, unique technology platform that ensures a high quality of analysis results and reliability. The instruments are known for their robustness and high precision as well as the easy to use yet advanced analysis capabilities.

Our primary focus is on cell counting and cell analysis, especially for use in life sciences research, clinical diagnostics and in production and quality control within the pharmaceutical industry.

COMECER GROUP

COMECER GROUP — Booth 707

Comecer is a confirmed leader in isolation technology for pharmaceutical, biotech, chemical, and food industry applications. Its portfolio covers various standard products, but also high-quality customised solutions thanks to specific expertise acquired over the years. Continuous technological updating and the particular client requirements contribute to ensuring operator safety and product sterility. Its containment solutions range from isolators that handle highly active principles or excipients to multi-stage isolators for chemical synthesis or laboratory operations.

Its asepsis offerings include isolators designed for sterility tests and formulation of sterile drugs, isolators or RABs integrated into filling lines, and dedicated solutions for Regenerative Medicine applications, including the innovative FlexyCult incubation system for Advanced Therapy labs.

Comecer products are known and used in hospitals, universities, research centers, pharmaceutical companies and large industrial groups worldwide.

COMPASS BIOMEDICAL

Compass Biomedical — Booth 521

Compass Biomedical is the recognized leader in blood component products and biologic solutions for cell culture, tissue repair, wound care, and ophthalmic therapies. In addition to a specialized suite of products, our experts provide product development consultation to help translate novel research and bring it to market.

Compass Biomedical's PLUS™, is a cytokine rich replacement for fetal bovine serum (FBS). PLUS™ supports the culture of various human cell types, including mesenchymal stromal cells (MSCs), adipose derived stem cells (ASCs), dendritic cells, dermal fibroblasts, epidermal keratinocytes, and endothelial cells. PLUS™

is available as research grade and GMP grade for the development of cell therapy products. Stop by booth 521 to learn more about Compass Biomedical's products and services.

COOK® REGENTEC

Cook Regentec — Booth 501

Cook Regentec creates enabling technologies and tools for cell-based therapies and cGMP bioprocessing. From concept to commercialization, our product lines cover expansion, packaging, storing, thawing, delivering, and processing.

CORD BLOOD ASSOCIATION

Cord Blood Association — Booth 704

The Cord Blood Association is an international, non-profit organization that promotes both public and family cord blood banking. Its mission is to save lives and change medicine.

Founded in 2014, the CBA has five priorities: advocacy for the cord blood community, quality products and services, market expansion, research and development, and public and professional education. The 2018 ISCT Annual Meeting in Montreal will be the fourth consecutive year in which CBA has co-sponsored a pre-conference workshop on cord blood technologies and therapies including hematopoietic reconstitution and regenerative medicine.

CBA membership is open to individuals within the cord blood community, banks, health care professionals, industry partners and the general public. Learn more about the association and how you can participate and become a member at the CBA exhibit booth.

CORNING

Corning Life Sciences — Booth 606

From microcarriers to advanced surfaces and stacked vessels, Corning's cell therapy portfolio allows you to efficiently expand and scale-up cells for research and production. Choose from hundreds of vessels, the widest variety of cell culture surfaces, and custom media in a variety of single use technology packaging configurations.

Cryo Bio System
IMV Technologies Group

Cryo Bio System — Booth 702

Founded in 1987, Cryo Bio System is the human division of IMV Technologies group.

Exclusively dedicated to Life Science and Biodiversity, Cryo Bio System strives to provide the IMV Technologies Group expertise for the cryopreservation of biological samples to scientists in all fields through a range of innovative High Security products for:

- Biorepositories, epidemiological research
- Blood transfusion Haemovigilance
- Regenerative medicine: Cell and genetic therapy, Stem Cells
- Medical and Pharmaceutical Research - Assisted Reproductive Technologies (A.R.T.)
- Genomics, Proteomics
- Medico-legal storage
- Military Medicine
- Biodiversity: Animal and Vegetal Germplasm conservation.

Our international network, through subsidiaries in the United States, Italy, the Netherlands, India, China and our distributors in over 70 countries, offers both high quality products and technical support to the professionals.

Custom BioGenic Systems

Custom BioGenic Systems — Booth 602

Founded in 1987, Custom Biogenic Systems is a global leader in the design and manufacture of state of the art liquid nitrogen laboratory freezers, cryogenic equipment and accessories. The CBS Isothermal Liquid Nitrogen Vapor Storage System was patented in 2000 and Custom Biogenic Systems continues to be an innovative leader in the design of cryogenic equipment and supplies. In addition to cryopreservation equipment, we supply upright freezer racks, chest freezer racks, liquid nitrogen freezer racks, canisters / cassettes and frames as well as laboratory boxes and dividers. By understanding the needs of the biotechnology industry, Custom Biogenic Systems provides superior laboratory freezers and complete inventory control rack systems that support veterinarian, horticulture, pharmaceutical, industrial, scientific and research laboratories and banks.

DIGI-TRAX®

Digi-Trax — Booth 402

Digi-Trax is the global leader in compliance labeling solutions in healthcare...we offer an extensive product line for the blood bank and cellular therapy markets – Our premier ISBT 128 bar code compliant systems include software, printers, scanners, validation, and comprehensive technical service and support. Our easy-to-use HemaTrax-CT cellular therapy labeling system can be found in many facilities worldwide, in both standalone and integrated

versions. As an annual HemaTrax- CT-LSS (lifecycle software support) subscriber, you can ensure that the labels you print for your cellular therapy products conform to the latest requirements. For years, Digi-Trax has been a significant participant on several ICCBBA committees. We have an ongoing commitment to positively influencing the direction of cellular therapy labeling.

DRAPER®

Draper Booth 618

Draper is an independent, not-for-profit corporation, which means its primary commitment is to the success of clients' missions rather than to shareholders. For either government or private sector customers, Draper leverages its deep experience and innovative thinking to be an effective engineering research and development partner, designing solutions or objectively evaluating the ideas or products of others. Draper will partner with other organizations – from large for-profit prime contractors, to government agencies, to university researchers – in a variety of capacities. Services Draper provides range from concept development through delivered solution and lifecycle support. Draper's multidisciplinary teams of engineers and scientists can deliver useful solutions to even the most critical problems.

eceptionist

Eceptionist, Inc. Booth 610

Eceptionist is a global software company offering a cloud-based SaaS platform that enables healthcare organizations to manage the request and referral of services across the healthcare economy. Supported by various administrative tools and our sophisticated workflow engine, Eceptionist customers can manage and coordinate the scheduling of resources, complex services, tasks, in-person and collaborative telehealth events. Eceptionist is comprised of four core modules – **Enterprise Scheduling, Triage and eReferral Manager, Telehealth Manager and Wait List Manager.** Each module may be implemented independently, in groups or all together for a seamless technology experience.

EMO 艾歐生物醫學股份有限公司 EMO BIOMEDICINE CORP

EMO Biomedicine Corporation Not Exhibiting

EMO Biomedicine Corporation (www.emobio.com) was founded in Taipei, Taiwan in 2004. In order to be the world leading company in the field of cellular therapy, EMO has developed necessary platform technologies related to manufacturing and analysis for therapeutic cell- and tissue-based products (HCT/Ps).

Since 2004, EMO has been providing comprehensive cell-based bioassays and Contract Research Services to local and global clients, including pharmaceutical and biotech companies.

After inspection by Taiwan FDA in 2010, EMO started to offer Contract Manufacturing Service of cell-based

products to support local and oversea clients who lack GTP/GMP compliant facilities and quality management systems. Existed testing services accompanied with manufacturing service, EMO offers a complete service system, including manufacturing and product characterization, for cell therapy clients.

Entegris PURE ADVANTAGE

Entegris Booth 423

For 50 years, Entegris has been dedicated to developing the purest products that gain you the greatest yield. Our passion for pure has enabled manufacturers to make smaller and faster microchips, eliminate costly passivation steps in drug manufacturing, and even improve thermodynamic efficiency in aircraft engines.

With a broad product offering, advanced manufacturing capabilities, worldwide infrastructure and unmatched technical expertise, our pure passion is your advantage.

eppendorf

Eppendorf Booth 425

By exploiting the strong synergies in bioreactor technology and polymer manufacturing, Eppendorf has emerged as a global player and valuable resource to its customers in the bioprocess marketplace.

With a comprehensive offering of single-use and traditional products for the growth of mammalian and microbial cells, and working volumes of 60 mL – 2,400 L, the Eppendorf bioprocess portfolio can satisfy the demands of process development through production.

Many labs trust Eppendorf as an expert partner for stem cell bioprocessing. Eppendorf fosters close relationships with researchers from the stem cell field all over the globe to fully understand their needs.

ESCO VACCIXCELL ADHERENT BIOPROCESSING SPECIALIST

Esco VacciXcell Booth 218

Esco VacciXcell is the bioprocessing division of Esco Group of Companies. We specialize in marketing and manufacturing bioprocessing equipment for adherent cell culture. VacciXcell's core technology, the tide motion system, is an ideal platform for high yield and low cost production of biologics like vaccines, mAb, protein, and stem cell for therapy among others. Moreover, we provide turnkey manufacturing solutions.

It is our primary vision to help developing nations be self-sufficient in the manufacturing, storing, distribution and administration of vaccines and other biologics. We reach out to our target market by utilizing Esco's presence in over 100 countries globally. It has always been our mission to provide our clients with a complete solution from Discovery to Delivery for all their bioprocessing needs. We do this by ensuring a high quality, efficient, dependable and cost effective product line.

Extract Technology — Booth 307

Extract Technology are a leading worldwide supplier of containment and aseptic systems for the pharmaceutical, healthcare, biotech and chemical markets.

With a well-established and prestigious international client portfolio built on a reputation for excellence and a culture of continual improvement we are recognised as one of the leading containment and aseptic manufacturers in the world.

Extract Technology have worked closely with a number of global organisations in providing cell therapy isolator systems delivering client specific solutions for their applications.

Taking this knowledge, experience and expert consultation we have developed and launched our new innovative modular isolator solution for cell and gene therapy processing which we are proud to be exhibiting for the first time at the ISCT.

Foundation for the Accreditation of Cellular Therapy — Booth 706

FACT is a non-profit corporation founded for the purpose of voluntary inspection and accreditation in the field of cellular therapy, cord blood banking, and regenerative medicine. The objective of FACT is to improve the quality of cellular therapy through peer-developed standards, education and accreditation for the benefits of patients.

FloDesign Sonics — Booth 619

FloDesign Sonics (FDS) is pioneering groundbreaking technology that fundamentally changes the way we capture, filter, separate, and concentrate particles in fluid. Without the need for filters, we are free to explore a world of exciting applications. FDS' patented acoustic wave separation platforms utilize macroscale acoustofluidics principles to accomplish a variety of cell processing applications such as clarification, perfusion, concentration, washing, acoustic affinity cell selection, and label-free cell selection. FDS recognizes that to move cell and gene therapy forward, manufacturers must develop robust, closed, and automated commercial processes. Engineers need bioprocess equipment that is flexible and adaptable to broad and evolving process needs. Solutions must also be modular so unit operations can be connected, combined, and scaled. Acoustics Cell Processing is a new tool that enables the critical transition from manual, high-touch, open manufacturing operations to an integrated commercial process. Gentle forces created using ultrasonic waves manipulate cells for a variety of unit operations such as concentrate-wash, affinity selection, or other separations.

Fresenius Kabi USA — Booth 419

Fresenius Kabi is a global healthcare company that specializes in lifesaving medicines and technologies for infusion, transfusion and clinical nutrition. Our products and services are used to help care for critically and chronically ill patients. Fresenius Kabi features the Lovo cell processing system (www.fresenius-kabi.us/lovo) that washes and concentrates white blood cells using spinning membrane filtration technology.

GE Healthcare — Booth 515

GE Healthcare Life Sciences provides expertise and tools for a wide range of applications, including basic research of cells and proteins, drug discovery research, and tools to support large-scale manufacturing of biopharmaceuticals. Our Cell Therapy business enables customers from pharmaceutical manufacturing as part of our Cell Processing segment, to clinicians and technicians in our Cell Banking and Point of Care segments to help meet immediate needs, as well as innovative production capabilities for the future.

GenCure — Booth 715

GenCure enables the development of cell and tissue based therapies and companies by providing access to source materials, biomanufacturing services and clinical research support. Leveraging our core resources and in-house expertise including GMP contract manufacturing, process development, quality management, compliance and testing, GenCure is expanding its focus and collaborating with outside partners to develop new approaches for cell expansion and manufacturing in the regenerative medicine field.

For more information, visit www.GenCureBiomanufacturing.org

GenesisBPS — Booth 321

GenesisBPS is a global manufacturer and supplier of precision equipment to clinical laboratories, life science customers, blood collection and cellular therapy organizations. Our product portfolio represents quality and consistency in a world of ever-changing requirements for collection, processing, safe storage and transport.

GenesisBPS is the one-stop-shop for all your blood bank and cellular laboratory needs. Call us to learn more about our Blood Collection Devices, Tube Sealers, Dry Plasma Thawers, Waferless Sterile Tube Welder's, Therapeutic Phlebotomy products, specialty blood bags and a whole lot more.

To learn more, visit www.GenesisBPS.com or call one of our representatives at 866-71-BLOOD. GenesisBPS...Technology is in our blood!

GOLD-SIM

GOLD SIM International Group — Booth 724

GOLD SIM International Group is a multinational, professional cell technology and cryogenic technology corporation. We have branches and R&D centers in USA, Europe and China. Since our establishment, GOLD SIM has been committed to continuous research and developments in bio-cryogenics, bio-bank, cell factory, automation and lab equipments. With our professional R&D team and strong financial strength, we have formed a comprehensive product range, such as vapor phase LN2 cryogenic storage system, sample management software, freeze dryer, CO2 incubator, hybridization oven, bioreactor, controlled rate freezer, auto cell culture system, auto cell separation system, auto cell expansion system, etc. Our service scope has been expanded to hospital biobank, cord blood bank, stem cell bank, cellular therapy company, cell factory, university, pharmaceutical company, etc.

HemaCare™

HemaCare Corporation — Booth 208

HemaCare is a leader in the customization of human-derived biological products and services for biomedical research, drug discovery, and cellular therapy process development. With its network of FDA-registered and GMP/GTP-compliant collection centers, extensive registry of repeat donors, isolation laboratory, and 40 years of expertise, HemaCare provides a variety of primary blood cells and tissues, and supports commercialization with apheresis collections, directly enabling customers to advance both autologous and allogeneic cellular therapies.

HÉMA-QUÉBEC

Héma-Québec — Canadian Pavilion

In addition to its role as a supplier of blood products, human tissues and mother's milk, Héma-Québec manages the Stem Cell Donor Registry in Quebec, operates the largest NetCord-FACT-accredited Public Cord Blood Bank in Canada and is an active member of the World Marrow Donor Association. En plus de son rôle de fournisseur de produits sanguins, de tissus humains et de lait maternel, Héma-Québec gère le registre des donneurs de cellules souches au Québec, exploite la plus grande banque publique de sang de cordon accréditée NetCord-FACT au Canada et est un membre actif de la World Marrow Donor Association.

ICCBBA

ICCBBA — Booth 325

ICCBBA is the not-for-profit, nongovernmental international standards organization in official relations with the World Health Organization responsible for the management and development of the ISBT 128 Standard. Used in more than 85 countries across six continents and disparate health care systems, ISBT 128 is the global standard for the terminology, identification, coding, and labeling of medical products of human origin including blood, cell, tissue, milk, and organ products. The Standard has been designed to ensure the highest levels of accuracy, safety, and efficiency for the benefit of donors and patients worldwide.

To learn more information or to submit inquiries, contact our help desk as iccbba@iccbba.org.

immudex

Immudex — Booth 601

Based in Copenhagen, Denmark, with North American operations based in Fairfax, Virginia, Immudex provides MHC Dextramers for the monitoring of antigen-specific T cells. Under an agreement with the US Cancer Immunotherapy Consortium (CIC) and the European Cancer Immunotherapy Consortium (CIMT), Immudex also provides MHC Multimer and Elispot proficiency panel services worldwide.

Immudex's MHC Dextramer® products are utilized for the quantification or sorting of antigen-specific T cells in life science research, in vitro diagnostics, as well as the development of immunotherapeutics and vaccines. The primary focus is research-use-only products for the immune monitoring of immunotherapy development, and monitoring of CMV cellular immunity in transplant and other immune-deficient patients. The 510(k) cleared and CE-marked Dextramer CMV Kit is used clinically for the quantification of CMV-specific T cells post stem cell transplant.

Our soon-to-be released DNA tagged Dextramer reagents enable massive multiplexing of antigen-specific T cell detection. To date we have achieved quantitation of over 1000 CD8 T cell specificities in a single, small blood sample. To find out more about how Immudex is improving immune monitoring and enabling better treatment decisions please visit us at www.immudex.com. To learn more about proficiency panel participation email proficiencypanel@immudex.com

**The Immudex CMV kit is for Research Use Only in the United States*

Instant™ SYSTEMS

Instant Systems — Booth 323

Instant Systems is an industry leader in custom, high-performance, flexible packaging for cellular therapy and pharmaceutical industries, providing complete packaging systems for processing, storage, transport, and delivery of biologics. Instant Systems' product portfolio has a proven record of excelling under the most

challenging conditions, while their rapid prototyping services minimize time to market. Instant Systems' LEAN and scalable production systems deliver in record time, world class quality and cost effective packaging systems to all market segments. To find out how our dedicated engineering teams can help develop packaging to maximize cell expansion and cell recovery please call 800-706-5808 or email info@instantsystems.com.

IrvineScientific

Irvine Scientific — Booth 303

Irvine Scientific, a member of the JXTG Group, has been at the forefront of cell culture media development for more than 45-years. Possessing an unrivaled heritage of innovation, superior quality, and technical expertise, the company supplies the cell therapy, immunotherapy, biopharmaceutical, cytogenetic, and Assisted Reproductive Technologies (ART) industries with a range of advanced cell culture media products and expert development, optimization, and commercial manufacturing services.

Through the PRIME®-XV media platform, Irvine Scientific delivers specialized cell culture media solutions to support basic, translational, and clinical research using both stem cells and primary cells.

JSRM

Japanese Society for Regenerative Medicine — Booth 324

JSRM, established in May 2001, is the largest society for regenerative medicine in the world, with approximately 6000 members involved in research in a wide variety of fields in the natural sciences such as basic medicine/dentistry, clinical medicine/dentistry, tissue engineering, and cell biology, as well as fields in the humanities and in sociology such as bioethics, regulatory science, law, and medical economics. The participating members come from various domains of academia, industry, and government, and JSRM is recognized as the only platform beyond institutional borders where they can engage in discussions regarding a host of challenges brought about by the new field of Regenerative Medicine. After 2016, JSRM acquired competitive funds from the Japan Agency for Medical Research and Development (AMED) and has since been able to utilize public funding for activities such as the support of clinical research, development of human resources, development of clinical research data systems, coordination of academia–industry translations, and interactive communication with patients/citizens, resulting in remarkable achievements including undertaking over 40 consulting engagements in support of clinical research and conducting educational workshops for most of the nationally accredited regenerative medicine committees. In particular, development of the "Good Post-marketing Study Practice" standard compatible database and "National Regenerative Medicine Database (NRMD)" is gaining attention as another global first in the attempt to acquire real world evidence from all clinical cases of regenerative medicine and cell therapy.

KBI BIOPHARMA

KBI Biopharma — Booth 709

KBI Biopharma, Inc. is a biopharmaceutical Contract Development and Manufacturing Organization that accelerates the development of innovative discoveries into life-changing biological products. From early-stage to academic/non-profit organizations, to many of the world's largest pharmaceutical companies, KBI has served 250+ clients globally to accelerate and optimize their drug development programs.

KBI's extensive track record of successful programs is a result of its unique approach: applying the insight gained from our advanced biophysical and analytical protein characterization techniques toward the development of robust and scalable processes. KBI delivers accelerated and integrated process development and cGMP manufacturing programs for a wide range of recombinant protein Active Pharmaceutical Ingredients (API) and cell therapy products for our clients. KBI was founded in 1996 and operates 4 facilities: Durham and Research Triangle Park (NC), Boulder (CO), and The Woodlands (TX).

KeyBiologics LLC

Key Biologics, LLC — Booth 403

Key Biologics, LLC (Key) was built upon assets of Lifeblood Biological Services, a wholly-owned subsidiary of the Mid-South Regional Blood Center (Lifeblood), that Dr. Edward Scott created in 1999 and led while serving as Lifeblood's CEO. He retired and purchased the subsidiary in 2009, renaming it Key Biologics, LLC.

Key provides biological materials to cellular therapy and regenerative medicine researchers around the U.S., in Europe, Asia and Australia. Products are collected from normal subjects under Key's proprietary, IRB-approved, protocols and used for pre-clinical research, process development, development of in vitro diagnostics, to support clinical trials, and for further manufacturing into commercial clinical products. Key also has an approved protocol for collection from patients with hematologic malignancy. All product collections and product manipulations are performed at Key's site in Memphis.

Products include whole blood and its components, apheresis-collected platelets and MNC products (with or without G-CSF administration). Patients' collections are non-mobilized. Plerixafor mobilization and bone marrow collections will be initiated in 2018.

Products are shipped without further processing or processed in accordance with a customer's requirements prior to shipment. Processing includes peripheral blood mononuclear cell (PBMC) separation from whole blood and other leukocyte-rich raw materials, and immuno-magnetic bead selection of cells.

Macopharma — Booth 615

Macopharma's Biotherapy division is specialised in the fields of cellular therapy and regenerative medicine. We provide our customers with solutions for Photopheresis treatment and systems to collect, process, store and expand cord blood cells.

MAK-SYSTEM — Booth 519

MAK-SYSTEM International Group, leading software provider for more than 34 years in the digital business for healthcare and a footprint in 65 countries, presents its advanced and modular software solution called T.C.S. at booth # 519.

The T.C.S. software, used by Processing Labs and Transplantation centers worldwide, is designed to accomplish complete traceability in process management of any Tissue & cell from procurement until infusion.

MaxCyte — Booth 309

MaxCyte's non-viral delivery platform allows for engineering of nearly all cell types, including human primary cells, with any molecule, at any scale for use in drug discovery and development, biomanufacturing, gene editing, cell therapy, and immuno-oncology. Its consistency and minimal cell disturbance facilitate rapid, clinical and commercial grade cell engineering.

McGill Stem Cell and Regenerative Medicine Network — Canadian Pavilion

McGill University is one of the leading institutions in the study of cellular therapeutics. In 2013, the McGill Stem Cell and Regenerative Medicine (SCRM) Network was initiated with the mission to increase inter-departmental, inter-faculty and clinical research collaborations within and outside the McGill University Community. With our new graduate program in SCRM, we aim to foster further partnerships with other universities and the private sector towards advancing the study and use of SCRM for the benefit of Canadians and beyond.

Le réseau en cellules souches et médecine regénérative de l'université McGill

L'Université McGill est une des institutions académiques phares dans l'étude des thérapies cellulaires. En 2013, le réseau en cellules souches et médecine régénérative de McGill (CSMR) a été établi avec la mission d'accroître les interactions scientifiques et applications cliniques entre les collaborateurs internes et externes de l'université McGill. Avec le nouveau programme d'étude graduée en CSRM, nos objectifs sont de soutenir des partenariats d'excellence avec d'autres universités et le secteur privé dans le but de promouvoir la recherche et l'utilisation des CSRM à l'international et pour le bénéfice des Canadiens.

Mediware — Booth 507

Mediware's Transtem™ cellular therapy software was built from the ground up to meet the unique challenges of cell therapy labs, BMT programs, cord blood banks, and regenerative medicine facilities. Transtem streamlines the needs of laboratory staffers and clinicians, allowing you to manage cellular products from collection through processing, storage and infusion – all in one, complete and comprehensive system – providing you with a 360-degree view of treatments, outcomes, and program management. **KnowledgeTrak™** further improves the efficiency of cellular therapy organizations by combining document management with accreditation, audit, and competency management in a single integrated solution that significantly improves regulatory compliance.

Mill Creek Life Sciences — Booth 523

Mill Creek Life Sciences is the first company to commercialize human platelet lysate for cellular therapy. Based in Rochester, Minnesota, Mill Creek Life Sciences has been involved in clinical cellular therapies for a decade. MCLS is dedicated to providing the highest quality products for the research and clinical community.

MilliporeSigma — Booth 222

MilliporeSigma, a leading science and technology company in life science, offers solutions that enable scientists to conduct life science research efficiently and economically. Our novel cell culture systems and characterization tools will meet your needs from stem cell research to cGMP manufacturing. Our highly validated, optimized products provide solutions to your cell culture challenges, so you have the time to focus on your research and clinical goals and help advance the science of cell therapy.

Miltenyi Biotec

Miltenyi Biotec — Booth 415

Miltenyi Biotec is a global provider of products and services that advance biomedical research and cellular therapy. Our innovative tools support research at every level, from basic research to translational research to clinical application. This integrated portfolio enables scientists and clinicians to obtain, analyze, and utilize the cell. Our technologies cover techniques of sample preparation, cell isolation, cell sorting, flow cytometry, cell culture, molecular analysis, and preclinical imaging. Our more than 25 years of expertise spans research areas including immunology, stem cell biology, neuroscience, and cancer, and clinical research areas like hematology, graft engineering, and apheresis. In our commitment to the scientific community, we also offer comprehensive scientific support, consultation, and expert training. Today, Miltenyi Biotec has more than 1,800 employees in 25 countries – all dedicated to helping researchers and clinicians around the world make a greater impact on science and health.

MVE — Booth 504

MVE, the leading innovative manufacturer of secure cryogenic storage, features a complete line of stainless steel freezers, aluminum vapor shippers, and nitrogen handling equipment. Chart MVE's stainless steel freezers achieve the longest hold time and lowest LN2 consumption of comparable freezers, with vial capacities ranging from 3,200 to 94,000.

Cryogenic shipping became more secure with the introduction of Chart MVE's newest shippers that provide savings on packaging, shipping costs, dry ice, and disposal. Ask us about our new Fusion self-sustaining freezer that does not require regular LN2 fills.

National Research Council Canada | Conseil national de recherches Canada

National Research Council of Canada — Canadian Pavilion

The National Research Council (NRC) is the Government of Canada's premier research organization supporting industrial innovation, the advancement of knowledge and technology development, and fulfilling government mandates. Our Human Health Therapeutics Research Centre works with collaborators to develop and biomanufacture biologics and vaccines against emerging infections, cancer, Alzheimer's disease, and other indications.

Nordmark Biochemicals

Nordmark Biochemicals — Booth 119

Nordmark Biochemicals offers enzymes for high-yield isolation of viable cells, including the world's first animal-free GMP grade products, Collagenase AF-1 GMP Grade and Neutral Protease AF GMP Grade. Affordable research grade collagenases can be used for protocol development before transitioning to the GMP grade alternatives with similar enzymatic activities for clinical cell isolation applications.

Nordmark Biochemicals Collagenase NB 6 GMP Grade is especially suitable for the high-yield isolation of viable stem cells in clinical applications. The pharmaceutical manufacturing standard guarantees stringent quality control and reliable lot-to-lot consistency. Supporting documentation and access to the drug master file registered with the FDA are available for GMP grade users.

Collagenase NB products were marketed and distributed worldwide exclusively by SERVA Electrophoresis GmbH until the end of 2017. In 2018, Nordmark, the world's largest manufacturer of collagenase, took over the distribution of Collagenase NB products in order to offer production, sales, marketing and support from one source.

NB Translational Enzymes: Research and GMP Grade Collagenase and Neutral Protease Enzymes - from Research to the Clinic.

Nordmark Biochemicals – Your reliable Collagenase NB partner

Northern Therapeutics Inc.

Northern Therapeutics, Inc. — Not Exhibiting

Northern Therapeutics Inc. is a private Canadian biopharmaceutical company committed to the discovery and development of novel cell and gene therapies to extend and enhance the quality of the lives of people suffering from chronic life-threatening pulmonary disorders, in the absence of toxicity and major side effects.

NOVARTIS

Novartis Oncology — Booth 115

At Novartis, our mission is to discover new ways to improve and extend people's lives. We use science-based innovation to address some of society's most challenging healthcare issues. We discover and develop breakthrough treatments and find new ways to deliver them to as many people as possible.

Ontario Institute for Regenerative Medicine — Canadian Pavilion

The Ontario Institute for Regenerative Medicine (OIRM) is a non-profit stem cell institute funded by the Ontario government and dedicated to transforming discoveries into clinical trials and cures. Through our commitment to collaboration and partnerships, we leverage our resources to fund and support promising advances. OIRM is a passionate champion for investigators and their patients as we build a healthier future for Ontario, Canada, and the world.

OriGen Biomedical — Booth 713

OriGen Biomedical manufactures a complete line of products for cryopreservation and cell culture for use in the fight against life-threatening diseases. We produce the PermaLife bag and Evolve bag in closed-system cell culture options, in a range of sizes. Our cell culture lines can be manufactured with any tube set you desire and employ current needle-free technologies. The CryoStore bag is designed for cryopreservation of stem cells in ultra low temperatures and is able to be used with a sterile dock device. We have an Overwrap available which has excellent low temperature freezing properties. We manufacture DMSO/Dextran in syringes and vials and we can create custom configurations to meet your labs needs. All of our products are CE-marked, and available in a variety of packaging configurations. To learn more about OriGen's products visit our website at www.origen.com.

PACT — Booth 318

NHLBI has funded 5 cell processing facilities; Baylor College of Medicine (CAGT), City of Hope (CBG), University of Minnesota (MCT), Moffitt Cancer Center, University of Miami (ISCI) and a Coordinating Center, The Emmes Corporation, to provide the services that were identified as essential for the advancement of cellular therapy research by the previous PACT External Advisory Panel, investigators using PACT services, NIH working groups, and NHLBI staff. These services include providing support for IND-enabling translational research, and diversifying the cell therapy expertise within the program. The cell processing facilities are committed to providing the investigator the data needed to support an Investigational New Drug (IND) application. The PACT Coordinating Center serves as the monitor and coordinator for organizational and regulatory aspects of the program. The cell processing facilities were selected to meet the needs of the program by diversifying the cell therapy expertise and permits the capability to manufacture and ship cell therapy products that are not feasible over lengthy distances between the facility and the requesting investigator.

Pall Life Sciences — Booth 109

Pall meets the demanding needs of customers discovering, developing and producing biologics, vaccines, cell and gene therapy products & classic pharmaceuticals. Pall is a leading provider of continuous bioprocessing, integrated automated systems and single-use solutions to pharmaceutical and biotechnology companies - from bioreactors, through downstream purification, to formulation and filling.

PBS Biotech — Booth 405

PBS Biotech creates innovative, robust, and scalable manufacturing platforms that enable the advancement of novel therapies for patients. We also leverage our unparalleled knowledge of scalable process development to provide world-class R&D services.

PeproTech, Inc. — Booth 420

Supporting Life Science Research since 1988, PeproTech is the trusted source for the developing and manufacturing of high quality cytokine products for the life-science and cell therapy market.

Over the past 29 years the company has grown into a global enterprise with state-of-the-art manufacturing facilities in the US, and offices around the world.

With over 2,000 products PeproTech has developed and refined innovated protocols to ensure quality, reliability and consistency.

Our mission is to provide the highest quality products and premium support that address the needs and demands of today's scientists and researchers.

We pride ourselves on being a trusted partner within the scientific community.

- Comprehensive line of Cytokines and Antibodies
- GMP Cytokines for Cell, Gene and Tissue Therapy
- Animal-Free Cytokines
- ELISA Development Kits
- Media Kits / Supplements

RegMedNet — Booth 221

RegMedNet is a community site that unites the diverse regenerative medicine community. Through partnership with our sister journal, Regenerative Medicine, we seek to educate and inspire to help this exciting field move forward at an even faster rate.

RENOLIT Medical — Booth 522

Renolit Medical designs and manufactures medical grade films, pellets, ports, caps, compounds and tubing for medical fluid handling and storage. These products are used in a broad range of medical and pharmaceutical applications and are produced in clean-room conditions under ISO 13485 certification. Our products are used for production of pharmaceutical packaging and medical devices like blood bags, bags for dialysis applications, bags for intravenous & parenteral nutrition solutions, biotechnology systems and infusion and transfusion sets.

Réseau Thécell Network — Canadian Pavilion

ThéCell is a non-profit network supported by the Fonds de recherche du Québec – Santé (FRQS) which is dedicated to further Cell, Gene and Tissue therapies. ThéCell mobilizes researchers and resources for the development of new therapeutic approaches in regenerative medicine and accelerates the translation of discoveries stemming from Quebec laboratories toward clinical applications and treatments. The network acts as a catalyst to promote the collaboration between more than 100 researchers, clinicians and their teams (over 160 students and 50 research professionals) involved in key health research areas (musculoskeletal and nervous systems, cardiovascular, pulmonary and renal systems, skin, cornea and gastrointestinal tissues, and hematology, oncology and immunology). Six infrastructures support the activities of the network including: preclinical, ethical and legal guidance as well as biobanking and GMP production facilities for cells and tissues used in clinical trials. ThéCell plays a central role in Quebec's regenerative medicine landscape by strengthening scientific exchanges and promoting the emergence of new partnerships.

Roche Diagnostics — Booth 624

Building on decades of experience as a diagnostics and pharmaceutical manufacturer, Roche CustomBiotech provides a broad range of products and services for diagnostics, life sciences and pharma biotech industry. Our extensive product portfolio spans the breadth of the industry, from enzymes and cofactors, substrates, customized kits and PCR-based kits based on rapid microbiological methods for Quality Control. Furthermore analyzers for cell growth monitoring and fermentation process monitoring are offered.

ROKIT — Booth 717

ROKIT is committed to bettering humanity through our contributions to the field of regenerative medicine.

We are strategically partnering with physicians and researchers around the world to create low-cost alternatives to current medical procedures.

We look forward to pushing the scientific and regulatory bounds of 3D printing from the bench to the clinic, starting right here in Korea and expanding around the globe.

With our accumulated expertise in 3D technology in the biomedical field and partnerships in clinical arenas, we believe we have come very close to bringing the future here.

RoosterBio Inc. — Booth 225

RoosterBio's stem cell tools blast open the most significant bottleneck in clinical trial initiation. RoosterBio is an innovative regenerative medicine technology company dedicated to driving the acceleration of stem cell-based therapeutics and supporting the development of a sustainable regenerative medicine industry. RoosterBio's high-volume, well-characterized adult hMSCs and paired high efficiency bioprocess media systems remove several years and millions of dollars from product development and clinical testing. This first-of-its-kind, "off the shelf" line of products, that enables customers to leap ahead in their own research and manufacturing, is revolutionizing how cell therapies and constructs that require living cells are developed, clinically-translated, and commercialized. Visit www.roosterbio.com

Saint-Gobain — Booth 603

Saint-Gobain Bioprocess Solutions is proud to take part in providing solutions for a multitude of cell therapy applications while collaborating with customers and industry partners to develop custom disposables, often for integration into automated systems. Through our material science expertise as well as our deep experience in bringing manufacturing technologies to scale, we are uniquely positioned to offer solutions to the numerous challenges faced by cell therapy manufacturers today.

Sartorius Stedim Biotech — Booth 616

As one of the world's leading providers of laboratory and process technologies, Sartorius Stedim Biotech empowers clients around the globe to implement complex and quality-critical processes in a timely and cost-effective manner for biopharmaceutical production and laboratory research. Sartorius has amassed a portfolio of innovative products and high quality services that are now being repurposed and refined to suit the emerging needs and intricacies of the regenerative medicine industry, as well as developing new technologies to accelerate the time to market. Recent acquisitions, such as Intellicyt for expedited high content flow cytometry, Virocyt for quantitative virus analysis and kSep for liquid phase separation and concentration, are all aimed to further enable and support research efforts. With its historic roots in advancing science and next generation technologies like single-use, Sartorius aims to play an impactful role in facilitating novel technology adoption and strengthening the translational pathways for these promising new therapeutics.

Savsu Technologies — Booth 718

Savsu Technologies builds integrated, innovative hardware and software solutions designed to protect live cell therapies during transport and storage. Our clients include cell and gene therapy companies, specialty couriers, biotech/biopharma, research institutions, governments, and NGOs.

Scinogy — Booth 404

The Scinogy team is dedicated to making your clinical results a commercial reality by providing optimized solutions for the development and manufacture of high quality, affordable cell therapies.

Choosing manufacturing technologies and a scale-up strategy for your therapy is a complex process. It is influenced by the decisions you have already made and the commercial and regulatory constraints within which your business must operate.

The culmination of 100 years of experience from more than 300 projects is enabling Scinogy to deliver manufacturing solutions that are revolutionizing the cell therapy industry.

Scinogy will soon be launching ROTEA, "an exciting new manufacturing technology that has the potential to reduce the cost and increase the efficiency of cell and gene therapies and RM based medicine. The anticipated size, cost and capability of Rotea make it ideally suited to process development and early stage clinical trials and its scalability should enable a seamless transition to commercial production" — Michael May, President & CEO, CCRM

Scinus Cell Expansion BV — Booth 711

Scinus Cell Expansion BV (Bilthoven, The Netherlands) is a privately owned, R&D-focused SME, founded in 2017 as a spin-out from Xpand Biotechnology BV. Our mission is to make cell therapies accessible for a global patient population. The core activity of Scinus is developing innovative bioreactor platform technology for the cell therapy industry. Our bioreactor technology offers a cost-effective, controlled alternative to conventional cell therapy production strategies. The patented technology allows cell therapy professionals to perform the entire cell expansion process, from biopsy to over 1 billion cells, within one closed system. Therefore, Scinus bioreactor technology is ideal for scale-out of both adherent and suspension cell culture, especially for autologous applications. We have the expertise to help cell therapy professionals transition to a commercial-scale production strategy for their cell therapy products.

Stem Cell Network — Canadian Pavilion

Supporting and building Canada's stem cell and regenerative medicine research sector has been the raison d'être of the Stem Cell Network (SCN) since its inception in 2001. Its work has been supported by the Government of Canada from the beginning.

In just over 16 years, SCN has forged a national community that has transformed stem cell research in Canada, brought research to the point where regenerative medicine is changing clinical practice, and established an outstanding international reputation. SCN has pushed the boundaries of what was a basic research area towards translational outcomes for the clinic and marketplace.

Le soutien et l'établissement du secteur de recherche sur les cellules souches et la médecine régénérative au Canada sont la raison d'être du Réseau de cellules souches (RCS) depuis sa création en 2001. Le gouvernement du Canada appuie les travaux du RCS depuis ses débuts.

En un peu plus de 16 ans, le RCS a mis sur pied une communauté nationale d'experts qui a transformé la recherche sur les cellules souches au Canada à un point tel que la médecine régénérative a maintenant révolutionné la pratique clinique, ce qui a permis au Canada d'acquérir une réputation exceptionnelle sur la scène mondiale. Le RCS a repoussé les limites de la recherche fondamentale si bien que les résultats translationnels qui en découlent mènent régulièrement à des applications cliniques et à la mise en marché de nouveaux produits.

STEM CELLS Journals — Booth 520

Publication in prestigious journals such as **STEM CELLS** and **STEM CELLS Translational Medicine** relies most heavily on the quality of data and the relevance of the subject. Stem Cells focuses on novel and mechanistic basic stem cell biology findings. Stem Cells Translational Medicine is dedicated to significantly advancing the clinical utilization of stem cell molecular and cellular biology by bridging stem cell research and clinical trials to improve patient outcomes.

StemBioSys, Inc. — Booth 620

StemBioSys, Inc. based in San Antonio, Texas, is a privately-held biomedical company focused on enhancing the growth and delivery of adult stem cells for research, therapeutic or drug discovery applications. Its patented and proprietary technology platforms – licensed from the UT Systems– overcome key obstacles to creating clinically useful stem cell therapies. The centerpiece of these enabling technologies is the HPME® (High Performance Micro Environment) platform. This cell derived 3-dimensional microenvironment allows a variety of stem cells to replicate more rapidly, maintain a small cell size and express markers indicative of retained stem cell potency beyond that seen with traditional tissue culture substrates. This year, StemBioSys will announce a new advanced version of its cell expansion technology and specialized cell lines enabled by this technology. Our products are available for the research market in the United States through VWR. We are actively seeking collaborations with partners looking for cell expansion technologies.

stemTrak — Booth 516

stemTrak provides configurable, web-based, data management software solutions that enable Blood and Marrow Transplant (BMT) Programs and Stem Cell Researchers to optimize their processes and centralize their data while eliminating errors, variance, and duplicative data entry. Our solutions empower physicians, scientists, health care professionals, and data managers to easily access critical program data in an intuitive user-friendly interface. stemTrak gives users an unparalleled ability to quickly and easily perform statistical analysis, respond to RFIs, and interface with NMDP, CIBMTR, and other agencies that require information for regulatory compliance. Our solutions provide flexibility and configurability while being fully compliant with and all Health Information Privacy rules and regulatory requirements established by the FDA. stemTrak has a profound knowledge of technology, patient care practices, and implementing software solutions in regulatory environments. Using this knowledge, stemTrak works with its clients to implement solutions that meet their requirements while exceeding their expectations.

Synex Consulting Ltd. — Booth 509

Synex was founded by Young Kim on 2002. In the initial stage, Synex provided regulatory affairs and reimbursement listing services for biologics, pharmaceuticals and medical device. After that, Synex has continuously expanded its services to provide customers with one-stop solution for health care business in Korea. Today Synex provided total solution for market access of healthcare products in Korea.

TECHNOFLEX — Booth 408

Over 40 years of experience have made Technoflex an innovative firm, as well as a European leader in the design and manufacture of flexible bags and connectors for the pharmaceutical industry.

As for cellular therapy, Technoflex provides state-of-the-art bags delivering high value in the domains of cryopreservation, or stem-cells culture.

Technoflex is proud to introduce SafeCell®, our new range of FEP bags for cell culture. Developed with a specific leak-proof design, SafeCell® offers optimal traceability. Recently Technoflex has released his new primary packaging Dual-Mix™ for lyophilizate and diluent.

Our proficiency in the pharmaceutical primary packaging of injectable drugs, allow us to produce in clean room ISO 7 (Class 10000) with a low AQL for supplying innovative containers. Cutting-edge technology and internal R&D resources enable us to provide all-inclusive solutions that are pioneering, reliable and easy-to-use. Technoflex also develops a high capacity working with all-kind flexible films, which may be subject to high thermal amplitudes and to permeability.

Terumo BCT — Booth 311

Terumo BCT is a global leader in blood component, therapeutic apheresis and cellular technologies. We believe in the potential of blood to do even more for patients than it does today. This belief inspires our innovation and strengthens our collaboration with customers.

Thermo Fisher Scientific — Booth 319

As the world leader in serving science, Thermo Fisher Scientific is uniquely positioned to provide the quality materials, services and support needed to accelerate the pace of advanced therapy

development. We understand the complexity of this rapidly evolving industry and partner with our customers as they transition from discovery to clinical research and commercial manufacturing. Our portfolio of trusted and recognized products and services, combined with over 50 years of cell culture expertise enables us to provide comprehensive solutions to support every step of the advanced cell therapy process – from cell collection through manufacture to clinical site delivery. Through our Thermo Scientific, Applied BioSystems, Invitrogen, Fisher Scientific, Fisher BioServices, Unity Lab Services, and Gibco Cell Therapy Systems (CTS) brands, we offer an unmatched combination of innovative technologies, purchasing convenience and comprehensive support. www.thermofisher.com.

ThermoGenesis

ThermoGenesis Corporation — Booth 322

ThermoGenesis Corporation, a wholly owned subsidiary of Cesca Therapeutics, is a pioneer and market leader in the development and commercialization of automated technologies for cell-based therapeutics and bioprocessing. The division's automated functionally-closed cell processing and cryopreservation systems provide researchers, physicians and cell-based manufacturers with solutions for Clinical Biobanking, Point-of-Care and Immuno-Oncology.

time:matters
A company of Lufthansa Cargo

time:matters — Booth 622

time:matters is specialized in worldwide transportation of urgent and sensitive commodities like cells and tissues for Cell & Gene Therapy. We have excelled also in accompanied HSC transports as well as international sample logistics. With our customers we develop vein-to-vein supply chains for their clinical trial and commercialized products. We provide solutions for temperature-controlled transports from cryo-preserved up until 37°C including return logistics of packaging. As handling partner for several airlines, we maintain access to your shipments even beyond airport gates and ensure special requirements like do not x-ray & upright loading first-hand.

Title 21 health solutions

Title21 Health Solutions — Booth 422

Title21 Health Solutions provides end-to-end data management solutions to support cell and gene therapy laboratories, regenerative medicine and BMT programs including clinical case management, laboratory processing, supplies and equipment management, product inventory management, ISBT 128 labeling, chain of custody, outcomes reporting and a full suite of quality management solutions. Title21 Health Solutions integrates with clinical information systems such as EHRs, interfaces with laboratory equipment, and is approved to submit and retrieve data via CIBMTR. Title21 reduces manual paperwork, improves efficiency and supports world-class patient care. Demo Title21's innovative technology in the Exhibit Hall at Booth #422!

TMC PHARMA

TMC Pharma Services — Booth 219

TMC Pharma Services (TMC) is a unique international Pharmaceutical Development Organisation (PDO) providing a full range of expert pharma/biotech support services.

TMC specialises in assisting clients with global development of advanced therapy and novel biotech products; often in rare/challenging indications.

TMC has been actively supporting clients developing cell/gene therapy products since 2009. Much of TMC's cell therapy work is truly trailblazing. For example, TMC helped a 'virtual' company developing an embryonic stem cell (ESC) therapy to obtain regulatory agencies' advice, successful clinical trial applications and completion of the first-ever ESC study in Europe.

We offer bespoke advice and services covering all aspects of product development, from the early discovery phase right through to marketing, including:

- Comprehensive regulatory activities
- Full-service clinical studies
- Manufacturing, importation and QP release
- Pharmacovigilance, Medical management
- Data management/statistics
- Medical writing
- Post-marketing support e.g. Licence holder, product distribution

We are hands-on problem-solvers who relish the fact that each project brings its own set of challenges. After assessing a client's needs, we assign a project-specific team with precisely the expertise needed, and that team drives the project through to successful completion.

Web: www.tmcpharma.com

Email: info@tmcpharma.com

TrakCel

TrakCel — Booth 407

TrakCel is the market leading designer, developer and deliverer of integrated technologies specifically created in 2012 to manage the international autologous and allogeneic cell, gene and immunotherapy supply chain. TrakCel's software platform has been developed in collaboration with, and increasingly adopted by leading companies in the cell, gene and immunotherapy industries. TrakCel's solutions deliver real-time control over the entire therapeutic supply chain, from sample collection through manufacturing to treatment delivery. The TrakCel platform accelerates global scale-up and scale-out of cell and gene therapy products, increasing efficiency and decreasing complexity, while maintaining needle-to-needle compliance and traceability.

TrakCel is headquartered in Cardiff, Wales, UK with US offices in California and New Jersey. It aims to employ over 100 people by end of 2019, following a number of senior appointments.

Vineti

Not exhibiting

Vineti creates innovative digital technology to drive the automation, production, and delivery of 21st-century medicine. The company combines leading software expertise with deep, first-hand industry experience in developing and commercializing personalized medicines to develop a cloud-based platform that ensures quality, scale, security, efficiency, traceability, and safety.

The Vineti platform provides actionable insights to continually optimize the cell and gene therapy process, accelerating time to revenue and decreasing costs. Caregivers and pharmaceutical pioneers are empowered to help more patients more effectively and safely, treatments are better understood and improved over time, and most importantly, there's an opportunity to provide greater health outcomes—and cures—to patients in need. Vineti supports a range of individualized therapies, including cell therapies, gene therapies, and personalized cancer vaccines, in clinical and commercial phases.

Vineti was co-founded by GE and the Mayo Clinic, and is 60 employees strong and growing. The company is based in San Francisco, California.

Waisman Biomanufacturing

Booth 121

Waisman Biomanufacturing has extensive experience in manufacturing a wide range of biotherapeutics for human clinical trials including gene therapeutics, cell therapeutics, vaccines, and recombinant proteins. Waisman has developed platform manufacturing processes and analytical methods to support clinical production of several classes of biotherapeutics including plasmid DNA, Mesenchymal Stromal Cells (MSCs), pluripotent stem cells (hESCs, iPSCs), neural progenitor cells, adenoviral vectors, AAV, and lentiviral vectors. We provide four key components for cellular therapies: providing reprogramming vectors (EBV plasmids), sourcing human cells in compliance with regulations for HCT/Ps, Tissues, and Cellular and Tissue-Based Products (21 CFR 1271), producing cell banks under cGMPs, and production of final cell therapeutics including differentiated cell types. Waisman provides full support for IND filings by providing QA audited batch records and support for your Investigational New Drug (IND) filing including Chemistry, Manufacturing, and Control (CMC) support and access to the Waisman Biomanufacturing Type V Facility Master File.

West Pharmaceutical Services

Booth 517

West Pharmaceutical Services, Inc. is a leading manufacturer of packaging components and delivery systems for injectable drugs and healthcare products. Working by the side of its customers from concept to patient, West creates products that promote the efficiency, reliability and safety of the world's pharmaceutical drug supply. West is headquartered in Exton, Pennsylvania, and supports its customers from locations in North and South America, Europe, Asia and Australia. West's 2016 net sales of $1.5 billion reflect the daily use of approximately 112 million of its components and devices, which are designed to improve the delivery of healthcare to patients around the world.

Wilson Wolf Corporation

Booth 617

Wilson Wolf Corporation designs and manufactures G-Rex (Gas permeable Rapid cell EXpansion) cell expansion devices that have rapidly become the production platform of choice for Adoptive Cell Therapy. By eliminating the need for active gas exchange, rocking or stirring, these robust devices yield a more consistent and cost effective cell product than conventional culture systems. The G-Rex technology consistently yields larger, maximum density cell populations that are achieved through shortened culture durations, no technician interventions or media exchange, and reduced overall media usage. Devices range from Research & Development through large scale, closed systems designed to meet the needs of commercial applications. Stop by our booth to learn more about this platform technology and our ability to provide customized solutions for your cell processing needs.

World Courier

Booth 424

World Courier is a global specialty logistics company that designs world-class logistics and supply chain programs in complete alignment with our customers' business goals. With 2,000+ associates in more than 140 offices across the globe, we offer solutions that instill confidence in the on-time, on-temperature delivery of critical products. When trust is absolutely essential, there's only one choice: World Courier. www.worldcourier.com

GLOBAL to LOCAL at ISCT 2018

Global to Local Directory

The Baylor College Center for Cell and Gene Therapy — GL2

The Center for Cell and Gene Therapy houses two Good Manufacturing Practices (GMP) facilities for the preparation of cell and gene therapy products for use in Phase I/II clinical trials. In addition there is a Quality Control Laboratory, that performs testing on products and the manufacturing environment, and a Flow Cytometry Facility for analysis of therapeutic cells. The staff have considerable experience in preparing a wide range of cell and gene therapy products in one of the largest academic GMP facilities in North America.

For more information visit:

https://www.bcm.edu/centers/cell-and-gene-therapy/services-manufacturing

Children's National Health System — GL10

Children's National Health System, based in Washington, D.C., has been serving the nation's children since 1870. Children's National is #1 for babies and ranked in every specialty evaluated by U.S. News & World Report including placement in the top 10 for: Cancer (#7), Neurology and Neurosurgery (#9) Orthopedics (#9) and Nephrology (#10). Children's National has been designated two times as a Magnet® hospital, a designation given to hospitals that demonstrate the highest standards of nursing and patient care delivery. This pediatric academic health system offers expert care through a convenient, community-based primary care network and specialty outpatient centers. Home to the Children's Research Institute and the Sheikh Zayed Institute for Pediatric Surgical Innovation, Children's National is one of the nation's top NIH-funded pediatric institutions. Children's National is recognized for its expertise and innovation in pediatric care and as a strong voice for children through advocacy at the local, regional and national levels.

Clinical Cell and Vaccine Production Facility (CVPF)/University of Pennsylvania — GL9

The Clinical Cell and Vaccine Production Facility (CVPF) of Penn Medicine is the first-of-its-kind to make bench-to-bedside translational medicine a reality. Our work has led to several first-in-human trials, including the first use of a lentiviral vector, the first infusions of zinc finger nuclease genome-modified cells, the first use of lentivirally-modified cells to treat cancer, and the first FDA-approved CART-T cell therapy. We manufacture cell and gene biotherapeutics targeted against adult and pediatric cancers, HIV, stroke, and other diseases or disorders. Our state-of-the-art facilities are FACT accredited, and the CVPF functions as an NCI-approved Penn Medicine Abramson Cancer Center Shared Resource. Since 1999, the CVPF has supported >30 FDA IND applications at Penn Medicine and external institutions, and the CVPF has produced nearly 3,000 cellular vaccines, which have been administered to more than 1,000 patients. In the past year, the CVPF has manufactured 127 clinical products and 28 vaccines. Being first matters and Penn Medicine has a proven record of being first when it comes to bringing pioneering new treatments to those in need; the CVPF continues this mission with first-in-human trials underway that will change the face of medicine. For more information, see our Clinical Trials page.

Comprehensive Cell Solutions — GL8

Comprehensive Cell Solutions™ provides an unaffiliated, neutral platform for your biotechnology and clinical trial needs. We seamlessly integrate your ideas with our world-class expertise to advance medicine from bench to bedside. Our customized approach and collaborative model leverage our range of products and services, clinical trials management experience, and regulatory and accreditation history to achieve your goals. This flexible strategy adapts to your unique needs, and drives projects from concept to completion at every stage of the process. Our mission is to provide solutions that bring you to the forefront of patient care. Comprehensive Cell Solutions™ formed from the translational research group at the New York Blood Center, and pairs this history of excellence with new, cutting edge techniques.

Cytotherapy, ISCT Official Journal GL7

The journal brings readers the latest developments in the fast moving field of cellular therapy in man. This includes cell therapy for cancer, immune disorders, inherited diseases, tissue repair and regenerative medicine. The journal covers the science, translational development and treatment with variety of cell types including hematopoietic stem cells, immune cells (dendritic cells, NK, cells, T cells, antigen presenting cells) mesenchymal stromal cells, adipose cells, nerve, muscle, vascular and endothelial cells, and induced pluripotential stem cells. We also welcome manuscripts on subcellular derivatives such as exosomes. A specific focus is on translational research that brings cell therapy to the clinic. *Cytotherapy* publishes original papers, reviews, position papers editorials, commentaries and letters to the editor. We welcome "Protocols in *Cytotherapy*" bringing standard operating procedure for production specific cell types for clinical use within the reach of the readership.

Dana-Farber Cancer Institute GL7

Founded in Boston in 1947, Dana-Farber Cancer Institute is world renowned for its leadership in adult and pediatric cancer treatment and research. We develop and disseminate innovative patient therapies and scientific discoveries throughout the world. The Cell Manipulation Core Facility (CMCF) is FACT accredited manufacturing lab that supports the adult and pediatric stem cell transplant programs at DFCI, Brigham and Women's Hospital and Boston Children's Hospital. The CMCF is also a core facility of the Dana-Farber Harvard Cancer Center and supports innovative cellular therapy programs at DFCI and all of our partner hospitals. CMCF supports the development of new cell-based therapies and clinical research studies designed to evaluate the safety and efficacy of these novel treatments as well as standard of care products for stem cell transplantation. All cell manufacturing procedures are performed in environmentally controlled ISO-7 cleanrooms according to current Good Manufacturing Practices for cell and tissue processing. Current services include complex processing of hematopoietic stem cells for autologous or allogeneic transplantation, genetic modification of hematopoietic stem cells for gene therapy applications, genetic or culture mediated modification of immune cell populations for adoptive cellular therapy and reprogramming to create autologous induced pluripotent stem cells.

Fred Hutchinson Cancer Research Center GL4

At Fred Hutchinson Cancer Research Center, home to three Nobel laureates, interdisciplinary teams of world-renowned scientists seek new and innovative ways to prevent, diagnose and treat cancer, HIV/AIDS and other life-threatening diseases. Fred Hutch's pioneering work in bone marrow transplantation led to the development of immunotherapy, and Hutch scientists continue to be thought leaders and innovators in the field. Fred Hutch's robust pipeline of translational therapies is cultivated and supported by over 300 faculty members, onsite vector manufacturing and cell processing facilities, stellar clinical care partners, and a commitment to innovation.

Miami School of Medicine GL5

The University of Miami and the Interdisciplinary Stem Cell Institute (ISCI) are committed to bring the most innovative clinical treatment from the bench to the patient's bedside. The Clinical Research Cell Manufacturing Program (CRCMP) accelerates this process by putting in place state of the art facilities to process/manufacture cells, used in advanced cellular therapies for the treatment of heart, hematological, neurological, bone, cancer, eye and other chronic, debilitating, or incurable diseases.

The CRCMP Facility within the ISCI, was recently awarded with PACT grant and is accredited for FACT Common Standards. ISCI, CRCMP is composed of two manufacturing areas: an environment controlled area or clean room to manufacture products under section 351 of the PHS Act (more than minimally manipulated products) and a Development Laboratory (DL) area dedicated to process/manufacture cellular products under section 361 of the PHS Act (minimally manipulated products) and used to develop and validate new processes. The CRCMP manufactures and processes the products following the standard practices regulated under current Good Manufacturing Practice, 21 CFR Part 210 (cGMP), Human Cells, Tissues, and Cellular and Tissue-Based Products, 21 CFR Part 1271 (HCT/Ps) and FACT-JACIE International Standards for Cellular Therapy.

Stanford MEDICINE | Laboratory for Cell & Gene Medicine

Stanford Laboratory for Cell and Gene Medicine (LCGM) GL3

The LCGM is a purpose built cGMP compliant biologics manufacturing facility for the preparation of cell and gene therapy products for use in pilot and Phase I/II clinical investigations. The facility is led by the Executive Director of Stem Cells and Cellular Therapeutics Operations for Stanford Healthcare, managed by experienced professionals with specific training in Good Manufacturing Practices. The LCGM staffing is organized into independent groups for Manufacturing, Quality Systems and Regulatory Affairs. The Manufacturing staff performs process development, qualification and manufacturing of all clinical batches of cell and/or gene therapy products. The Quality Systems staff is responsible for document creation and control, raw materials, in process and final materials management as well as performing or managing analytic testing and final product release. The Quality Systems staff also ensures compliance with cGMP requirements for equipment, facilities and personnel. The Regulatory Affairs staff is responsible for creating and maintaining regulatory documentation on investigational products according to federal, state, local or institutional requirements and supports IND development and master file creation and maintenance for the LCGM facility and Stanford Cell and Gene Therapy products.

Tulane University SCHOOL OF MEDICINE

Tulane University GL6

Regenerative Medicine is a primary research focus of Tulane University and the Tulane National Primate Research Center, with areas of emphasis including: Cell Therapy (Adult Stem Cells), Tissue and Organ Engineering, and Gene Therapy. Tulane scientists have extensive experience with developing and testing of novel therapies on Cardiovascular Health & Complex Chronic Conditions (i.e. Hypertension, Diabetes, etc.); Neurodegenerative diseases; Pulmonary diseases; Infectious Diseases; Wounds; and Oncology. Our scientists have expertise with in vitro assays and a range of in vivo models including rodent and non-human primate animal models, as well as in human clinical trials - and have collaborations spanning the entire globe, with all types and sizes of for-profit and not-for-profit organizations. We are highly motivated to continue our emergence as a modern research University as we continually evolve our new paradigm for strategic alliances to best serve the needs of our collaborators.

PACT | Program for Advanced Cell Therapy DISCOVER • DEVELOP • DEPLOY

The University of Wisconsin Program for Advanced Cellular Therapy GL1

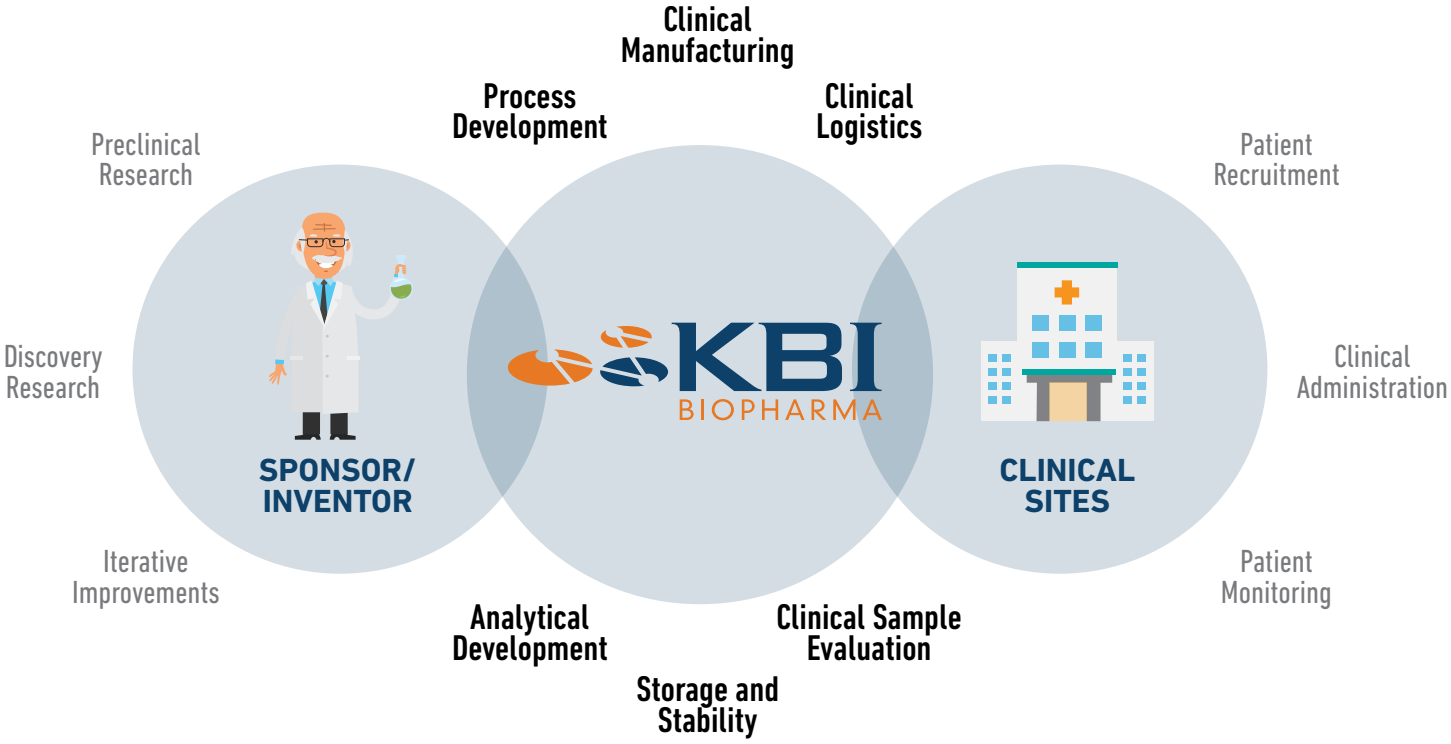
The University of Wisconsin Program for Advanced Cellular Therapy (PACT) aims to conduct first-in-human clinical trials to de-risk various aspects of advanced cell therapy. Our vision is to become the national leader in healthcare integrated personalized cell technologies for improving health outcomes in children and adults with unmet medical needs. Our GMP lab is intentionally embedded in the hospital service line so we can easily conduct autologous and allogeneic cell manipulation and deliver product/cells to subjects enrolled in UW Health clinical trials. We strive to develop healthcare and hospital-friendly solutions for cutting-edge cell innovations and devices that will enable FDA CBER INDs for cell technologies in cancer, immunotherapy and other medical conditions. The PACT GMP Cell Manufacturing facility is a state-of-the-art facility, equipped with partially and fully automated closed cell processing units and all other necessary equipment that is continuously monitored. It is ideally suited for production of GMP or “351” products that are more than minimally manipulated and held under Investigational New Drug status with the FDA.

KBI BIOPHARMA

A NEW PARADIGM IN THERAPY DESERVES A NEW PARADIGM IN PARTNERSHIPS

Your partner for cell therapy development and manufacturing.
Perfecting Processes. Manufacturing Miracles.

- Process Development
- Cell Therapy Manufacturing
- MCB and WCB expansion and storage
- Clinical trial specimen logistics
- Comparability studies
- Formulation, shipping and stability studies
- Assay creation and validation
- Quality control testing and support



INTRODUCING THE FUTURE OF ISCT

International Society

ISCT

Cell & Gene Therapy

For over 25 years, ISCT has connected the global cell and gene therapy community and advanced the translation of these innovative treatments for the benefit of patients worldwide.

CONNECT. COMMUNICATE. TRANSLATE.

THE FUTURE IS HERE.

JOIN ISCT TODAY!

celltherapysociety.org

ISCT EUROPE 2018 REGIONAL MEETING

SUBMIT YOUR ABSTRACTS BY MAY 22

REGISTER BY JUNE 19 FOR EARLY RATES

SEPTEMBER 12-14 FLORENCE, ITALY

Firenze Fiera Congress and Exhibition Center

www.isct-eu2018.com

International Society
ISCT
Cell & Gene Therapy
EUROPE REGION