Accelerating Cell & Gene Therapy Adoption: Proof of Concept to Standard of Care

PROGRAM
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A WARM WELCOME TO PARIS – VIRTUAL!

Although the internet has been around for decades, it’s only recently that virtual meetings have been at the forefront of everyone’s minds – and for good reason. ISCT 2020 PARIS VIRTUAL represents an unprecedented opportunity in a time of crisis, as we continue to stand strong in the face of COVID-19. Just as email has mostly replaced snail-mail, ISCT has initiated an accelerated embrace of virtual space to connect members of our truly global society.

From the Organizing Committee’s remarkable resilience in turning things around at breakneck speed, to the dedication of our Head Office, to the overwhelming enthusiasm of our membership registering from all around the world, ISCT has done its best to assemble a superb showcase of the best in cell and gene therapy. ISCT 2020 Paris Virtual promises to be a meeting like none before. The eyes of our peers are on us as we have re-imagined a focused program that I’m simply thrilled to introduce here.

We have re-imagined our annual meeting in virtual space! ISCT is taking a leading role in re-designing the program from the ground up. We will provide the same, much-loved sessions that are familiar to regular attendees of every ISCT Annual Meeting. I promise you will enjoy the highest quality education, discussion, and inspiration on the most timely and critical topics in our field today. Our roster of speakers is a handpicked group of key opinion leaders, and our program sessions have undergone peer review, development, and now optimization for the digital sphere.

We are presenting over 30 sessions, with over 50 hours of LIVE and on demand streaming. Live discussion periods are scheduled for many of these. Each of our 6 plenaries concentrates on the latest advances in basic research, pre-clinical studies and clinical trials for therapeutic modalities including MSCs, immune effector cells, exosomes, gene-engineered cells, and iPSCs. Daniel Weiss, our Chief Scientific Officer, has rallied together an exceptional Showcase on COVID-19, featuring reports from the front lines, and a deep dive into how our field is contributing to the fight against
the pandemic. Two of our pre-conference tracks (Cord Blood & Global Regulatory Perspectives) have been transformed into Hot Topic sessions, and we are bringing vital and practical information on Strategies for Commercialization, and Quality and Operations.

If you haven’t already visited our virtual conference tour, just think of Google Street View for cell and gene therapy sightseers. Wrapped around all this amazing scientific content is a highly interactive – and exciting – virtual reality platform. The ISCT 2020 Paris Virtual site houses an interactive exhibition hall, presentation theatres, an abstract e-poster hall with over 500 submitted abstracts, a partnering forum designed to set up one-on-one and group meetings, and much more. As with every Annual Meeting before, there is a sense of exploration, fun and opportunity for discovery!

On our platform, you will encounter over 1800 fellow attendees navigating throughout the two days of our Meeting. You will see booths and products designed for the web to introduce our sponsors and corporate exhibitors. You will find a virtual environment designed for comfortable, flexible, global access. Never before has it been easier to find what you’re looking for at an ISCT Annual Meeting – especially while sitting in your favourite chair.

Our Annual Meeting this year will also – as you’ve no doubt seen in our communications so far – endure beyond the live event. For twelve months, until May 2021, our annual meeting will function as a fully accessible and living archive. You can come back to this content, review and replay, comment alongside peers, and follow up with presenters for an unprecedented timeframe.

I am so honoured to be introducing this all to you – and I am deeply grateful for the hard work of our peers and friends that has driven this meeting to fruition.

Our ISCT 2020 Paris Virtual sponsors are diverse and numerous. I thank each of our Sponsors for supporting our vision of a virtual meeting with unusually short notice. To our friends at Maxcyte, Cytiva, and Penn Medicine – our diamond sponsors – I am humbled by your belief in what ISCT has to offer even in unprecedented terrain. I offer a heartfelt thank you. This year has been something of a roller coaster for everyone, but now we plan to enter calmer waters as we together sing “it’s a small world after all”.

Our ISCT 2020 Paris Virtual Organizing Committee has been tireless in reworking and adapting our Paris program into the inspiring suite it is now. I want to offer profound thanks on behalf of ISCT to the whole committee – and special thanks on top of this to Christian Chabannon, Rachele Cicciocippo, and Ivan Martin. In just weeks they have spearheaded a resilient and flexible approach to adapting our Annual Meeting plans that typically take more than two years to refine. The success of ISCT 2020 Paris Virtual will be a testament to their strong commitment under challenging circumstances – no one originally signed up to organise an international meeting during a pandemic.

I’ve always seen ISCT as a beacon to everyone involved in cell and gene therapy. At this moment, as I approach the end of my Presidential term, I have no doubt that our Society will continue to attract and showcase the best of the best.

I know this is a meeting that will inspire, inform, and connect us as a Society. I hope that all of you find that this offers a beautiful moment to enjoy in the troubling times that we have been withstanding every day. So please get your avatars activated – because soon we will be virtually standing face-to-face!

John Rasko, AO, BSc(Med), MBBS(Hons), PhD, MAICD, FFSc(RCPA), FRCPA, FRACP, FAHMS
ISCT President, 2018-2020
WELCOME TO ISCT 2020 PARIS VIRTUAL

On behalf of the ISCT 2020 Organizing Committee, we would like to extend a warm welcome to ISCT 2020 PARIS VIRTUAL! As the leading global Society focused on the translation of cell and gene therapy, our mission remains as important as ever in the face of these uncertain times. The CGT field shows immense resilience as it rapidly evolves, and so must ISCT. We, together with our colleagues on the Organizing Committee and ISCT Head Office, are thus excited to showcase scientific excellence and innovation as we Accelerate Cell & Gene Therapy Adoption: From Proof of Concept to Standard of Care through the inaugural ISCT Virtual Meeting. We are committed to bring you an unforgettable conference that will mix scientific excellence with pragmatism in bringing innovations to the market and to patients. This, of course, alongside friendship among peers across our global community.

Leveraging state-of-the-art virtual meeting technology, novel preclinical and translational science from 6 leading-edge areas of the CGT field will be showcased throughout the live broadcasted Plenary Sessions and associated Plenary Satellites. In particular, our Plenary Sessions will shine a spotlight on recent developments in the clinical application of MSC, CAR NK, and CAR T therapies, emerging gene-editing approaches for monogenetic and rare diseases, and the latest advancements in our understanding of extracellular vesicles and pluripotent cells as novel therapeutic agents. Unique to ISCT 2020 Paris Virtual, the program includes 2 feature sessions on the COVID-19 pandemic highlighting the current understanding of COVID-19 pathogenesis, individual reports from the front lines, supply chain issues, and current MSC and Immunotherapy treatment approaches.

A focal point of the Annual Meeting program remains the much anticipated Quality & Operations and Strategies for Commercialization Tracks, while a variety of other on-demand workshops will offer delegates greater insight into emerging issues, breaking news, and application specific considerations in the field of translational cell & gene therapy. Furthermore, ISCT 2020 witnessed a record-breaking number of abstract submissions which we are pleased to feature throughout the 48 hours of non-stop conference programming by way of both oral abstract presentations and an interactive E-Poster Hall.

Central to the execution of this high-caliber and comprehensive program is the valued support of our many industry partners who have sponsored the event; we are exceedingly thankful for their support. At the heart of the virtual meeting platform is our Exhibit Hall, where many of our partner organizations will be participating at customized exhibit booths. Be sure to visit the Exhibit Hall in between live broadcasted sessions and find the pulse of ISCT 2020’s next generation networking opportunities. Make sure to also check out the Corporate Symposia, Tutorials and Global Showcase Presentations to be featured across
both days of programming and stay up to date on the latest clinical research and product launches within the industry community.

Finally, we wish to extend our sincere gratitude to the dedicated and enthusiastic members who have contributed to the organization of this highly curated program. A huge thank you must also be extended to the incredible line-up of ISCT 2020 speakers who have graciously agreed to share their expertise and experiences during the virtual meeting. As a truly global Society we are excited to provide this highly interactive means of conferencing that will connect our members from all corners of the globe like never before. We hope you all enjoy your time together and look forward to a spectacular 2 days of digital age knowledge sharing, networking, and catching up with old friends.

Although we will not be able to share a coffee or your favorite drink with you in beautiful Paris, we would like to take this opportunity to reassure you that our community thrives with mutual respect and friendship. We will be waiting for the next editions of the ISCT Meeting in a conventional format, and in the meantime we wish you and your families, friends and teams to remain safe while facing these difficult times.

Sincerely,

Christian Chabannon, MD, PhD
ISCT 2020 Paris Co-Chair
Institut Paoli Calmettes and Aix-Marseille Université, FRA

Rachele Ciccioccioppo, MD
ISCT 2020 Paris Co-Chair
University of Verona, ITA

Ivan Martin, PhD
ISCT 2020 Paris Co-Chair
University Hospital Basel, CHE
ORGANIZING COMMITTEE

Meeting Co-Chairs

Christian Chabannon, MD, PhD
Institut Paoli Calmettes and Aix-Marseille Université
Marseille, France

Rachele Ciccocioppo, MD
University of Verona
Verona, Italy

Ivan Martin, PhD
University Hospital Basel
Basel, Switzerland

Organizing Committee Members

Gloria Carmona, MPharm
Co-Chair – Quality & Operations Track
Andalusian Network for Design and Translation of Advanced Therapies
Seville, Spain

Simon Ellison, MBA
Co-Chair – Strategies for Commercialization Track
World Courier
London, United Kingdom

Bernd Giebel, PhD
University Hospital Essen
Essen, Germany

Massimiliano Gnechi, MD, PhD, FESC
University of Pavia & IRCCS Policlinico San Matteo
Pavia, Italy

Ulrike Kohl, PhD
Fraunhofer IZI Leipzig; Institute of Clinical Immunology, University Leipzig
Leipzig, Germany

Mark Lowdell, PhD, FRCP, FRSB
Royal Free Hospital & University College London
Essex, United Kingdom

George Muschler, MD, PhD
Cleveland Clinic
Cleveland, OH, United States

Paula Salmikangas, PhD
Co-Chair – Quality & Operations Track
NDA Advisory Services Ltd.
Klaukkala, Finland

Sandeep Soni, MD
Stanford University
Palo Alto, CA, United States

Anthony Ting, PhD
Co-Chair – Strategies for Commercialization Track
Athersys, Inc.
Cleveland, OH, United States

Sowmya Viswanathan, PhD
University Health Network
Toronto, ON, Canada

Bruce Levine, PhD (ex-officio)
University of Pennsylvania
Philadelphia, PA, United States

John Rasko, AO, BSc(Med), MBBS(Hons), PhD, MAICD, FFSc(RCPA), FRCPA, FRACP, FAHMS (ex-officio)
University of Sydney
Sydney, Australia

Daniel J. Weiss, MD, PhD (ex-officio)
University of Vermont School of Medicine
Burlington, VT, United States
# ORGANIZING COMMITTEE

## Strategies for Commercialization Track Subcommittee

<table>
<thead>
<tr>
<th>Name</th>
<th>Position, Affiliation</th>
<th>Location</th>
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<tbody>
<tr>
<td>Simon Ellison</td>
<td>MBA, Co-Chair, World Courier</td>
<td>London, United Kingdom</td>
</tr>
<tr>
<td>Gerhard Bauer</td>
<td>PhD, UC Davis</td>
<td>Sacramento, CA, United States</td>
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<tr>
<td>Miguel Forte</td>
<td>MD, PhD, Bone Therapeutics, Rixensart, Belgium</td>
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<tr>
<td>Siofradh McMahon</td>
<td>MSc, CCRM</td>
<td>Toronto, ON, Canada</td>
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<tr>
<td>Julie Murrell</td>
<td>PhD, MilliporeSigma, Bedford, MA, United States</td>
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<tr>
<td>Julie Allickson</td>
<td>PhD, MS, MT(ASCP), Wake Forest Institute for Regenerative Medicine</td>
<td>Winston-Salem, NC, United States</td>
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<tr>
<td>Dominic Clarke</td>
<td>PhD, HemaCare, Winston-Salem, NC, United States</td>
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<tr>
<td>Antonio Lee</td>
<td>PhD, MEDIPOST, Rockville, MO, United States</td>
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<tr>
<td>Ruth McDermott</td>
<td>PhD, MBA, Sartorius Stedim Biotech, Hertfordshire, United Kingdom</td>
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<tr>
<td>Patrick Rivers</td>
<td>Aquilo Capital, San Francisco, CA, United States</td>
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<tr>
<td>Dolores Baksh</td>
<td>PhD, Cytiva, Boston, MA, United States</td>
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<tr>
<td>Mark Flower</td>
<td>Cellares, San Francisco, CA</td>
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<tr>
<td>Michael May</td>
<td>PhD, CCRM, Toronto, ON, Canada</td>
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<tr>
<td>William Milligan</td>
<td>Slemenent Biotherapeutics, Vancouver, BC, Canada</td>
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<tr>
<td>Anthony Ting</td>
<td>PhD, Co-Chair, Athersys, Cleveland, OH, United States</td>
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## Quality and Operations Track Subcommittee

<table>
<thead>
<tr>
<th>Name</th>
<th>Position, Affiliation</th>
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<tbody>
<tr>
<td>Gloria Carmona</td>
<td>MPharm, Co-Chair, Andalusian Network for Design and Translation of Advanced Therapies</td>
<td>Seville, Spain</td>
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<tr>
<td>Medhat Askar</td>
<td>MD, PhD, FRCPath, Baylor University Medical Center, Dallas, TX, United States</td>
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<tr>
<td>Shirley Bartido</td>
<td>PhD, MBA, Collectix, New York, NY, United States</td>
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<tr>
<td>Rosemarie Bell</td>
<td>B.App.Sc Micro/Biochem MASM, QCen Cell Therapeutics, QIMR Berghofer Medical Research Institute</td>
<td>Queensland, Australia</td>
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<tr>
<td>Mo Heidaran</td>
<td>PhD, Paresel International, Washington, DC, United States</td>
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<tr>
<td>Paula Salmikangas</td>
<td>PhD, Co-Chair, NDA Advisory Services Ltd., Kluuokkala, Finland</td>
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<tr>
<td>Emily Hopewell</td>
<td>PhD, MT, Indiana University School of Medicine, Indianapolis, IN, United States</td>
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<tr>
<td>Bangon (Day) Longsomboon</td>
<td>MA, University of Miami, Coral Gables, FL, United States</td>
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<tr>
<td>Gerry McKiernan</td>
<td>Cell Therapies Pty Ltd, Melbourne, Australia</td>
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<tr>
<td>Craig Wright</td>
<td>MSc, Royal Prince Alfred Hospital, Sydney, Australia</td>
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<tr>
<td>Shiraz Ziya</td>
<td>PhD, Sartorius Stedim Biotech, Staffordshire, United Kingdom</td>
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The Board of Directors

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<thead>
<tr>
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<tbody>
<tr>
<td>John Rasko</td>
<td>AO, BSc(Med), MBBS(Hons), PhD, MAICD, FFSc(RCPA), FRCPA, FRACP, FAHMS</td>
<td>Chair, President</td>
</tr>
<tr>
<td>Bruce Levine</td>
<td>PhD</td>
<td>President-Elect</td>
</tr>
<tr>
<td>Catherine Bollard</td>
<td>MBChB, MD</td>
<td>Chair, Strategic Advisory Council</td>
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<tr>
<td>Lizette Caballero</td>
<td>BS, MT(ASCP)</td>
<td>Global Secretary</td>
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<tr>
<td>Emily Hopewell</td>
<td>PhD</td>
<td>Interim Global Treasurer</td>
</tr>
<tr>
<td>Oscar Lee</td>
<td>MD, PhD</td>
<td>Asia, Regional Vice-President</td>
</tr>
<tr>
<td>Ngaire Elwood</td>
<td>PhD</td>
<td>Australia &amp; New Zealand, Regional Vice-President</td>
</tr>
<tr>
<td>Patricia Rocco</td>
<td>MD, PhD</td>
<td>South &amp; Central America, Regional Vice-President</td>
</tr>
<tr>
<td>Karen Nichols</td>
<td>Esq.</td>
<td>Chief Regulatory Officer</td>
</tr>
<tr>
<td>Jeannette Bloom</td>
<td>MBA, MT(ASCP), SBB</td>
<td>Elected Member Technologist</td>
</tr>
<tr>
<td>Queenie Jang</td>
<td>BSc (Pharmacy), MBA</td>
<td>Chief Executive Officer</td>
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Executive Management Committee

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<thead>
<tr>
<th>Name</th>
<th>Position</th>
<th>Term</th>
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<tbody>
<tr>
<td>Queenie Jang</td>
<td>BSc (Pharmacy), MBA</td>
<td>Chair, Chief Executive Officer</td>
</tr>
<tr>
<td>John Rasko</td>
<td>AO, BSc(Med), MBBS(Hons), PhD, MAICD, FFSc(RCPA), FRCPA, FRACP, FAHMS</td>
<td>President</td>
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<td>Chair, Strategic Advisory Council</td>
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<td>Emily Hopewell</td>
<td>PhD</td>
<td>Interim Global Treasurer</td>
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Strategic Advisory Council

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<tr>
<th>Name</th>
<th>Position</th>
<th>Term</th>
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<tbody>
<tr>
<td>Catherine Bollard</td>
<td>MBChB, MD</td>
<td>Chair, (Immediate Past President)</td>
</tr>
<tr>
<td>John Rasko</td>
<td>AO, BSc(Med), MBBS(Hons), PhD, MAICD, FFSc(RCPA), FRCPA, FRACP, FAHMS</td>
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<td>PhD</td>
<td>President-Elect</td>
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<tr>
<td>Queenie Jang</td>
<td>BSc (Pharmacy), MBA</td>
<td>Chief Executive Officer</td>
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<tr>
<td>Robert Negrin</td>
<td>MD</td>
<td>Member</td>
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<tr>
<td>Massimo Dominici</td>
<td>MD</td>
<td>Member</td>
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<tr>
<td>Keith Thompson</td>
<td>MSc, MBA</td>
<td>Member</td>
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<tr>
<td>Bryan Choi</td>
<td>PhD</td>
<td>Member</td>
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<tr>
<td>Sarah Nikiforow</td>
<td>MD, PhD</td>
<td>Member</td>
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<tr>
<td>John Barrett</td>
<td>MD</td>
<td>Member</td>
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## ISCT 2020 PARIS VIRTUAL OPENINGS

### DAY ONE: MAY 28

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Details</th>
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<tbody>
<tr>
<td>08:00-10:00</td>
<td><strong>ISCT PRESIDENT AND MEETING CO-CHAIRS WELCOME</strong></td>
<td>Live</td>
</tr>
<tr>
<td>10:00-11:00</td>
<td><strong>PLENARY SESSION – ADVANCES AND EMERGING TECHNOLOGIES IN IMMUNE-MEDIATED CANCER TREATMENTS</strong></td>
<td>Live</td>
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<tr>
<td>11:00-12:00</td>
<td><strong>CORPORATE SYMPOSIUM HOSTED BY MILTENYI BIOTEC – ADVANCED SOLUTIONS FOR CUTTING-EDGE CELL THERAPIES</strong></td>
<td>Pre-Recorded</td>
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<tr>
<td>12:00-14:00</td>
<td><strong>PLENARY SESSION – MESENCHYMAL STROMAL CELLS: FULL CIRCLE FROM BASIC RESEARCH INSIGHTS TO CLINICAL TRIAL UPDATES</strong></td>
<td>Pre-Recorded</td>
</tr>
<tr>
<td>14:00-16:00</td>
<td><strong>CORPORATE SYMPOSIUM HOSTED BY MILTENYI BIOTEC – IPSC FOR CARDIOVASCULAR REGENERATIVE MEDICINE AND DISEASE MODELLING</strong></td>
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<td>16:00-18:00</td>
<td><strong>PLENARY SESSION – IPSC FOR CARDIOVASCULAR REGENERATIVE MEDICINE AND DISEASE MODELLING</strong></td>
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<tr>
<td>18:00-20:00</td>
<td><strong>PLENARY SESSION – GENE THERAPY CLINICAL TRIALS</strong></td>
<td>Pre-Recorded</td>
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<td>20:00-22:00</td>
<td><strong>PRESEASON PLENARY – MASTERING PLURIPOTENT CELLS WITH THERAPEUTIC INTENT</strong></td>
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### Additional Information:

- **24/7 Virtual Partnering Forum Open**
- **Pre-Recorded Sessions**
- **Exhibit Hall**
- **Poster Hall**
- **Networking Interactive**
- **Virtual Hall**
- **PLUS MORE THAN 30 SESSIONS AND 50 HOURS OF EDUCATION**

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**ISCT President and Meeting Co-Chairs Welcome**

**Chair:** Sandeep Soni

**Chair:** Caroline Blumer-Toti

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**PLENARY SESSION – Advances and Emerging Technologies in Immune-Mediated Cancer Treatments**

**Chair:** Sowmya Viswanathan

**Speakers:**
- Massimiliano Gnecchi
- Katy Rezvani
- Halvard Bønig
- Paul Frenette
- Dominiqe Forge
- Loic Fieschi
- Pluripotent Stem Cells-Derived Endothelial Cells
- Use of Pluripotent Stem Cells for Regenerating/Repairing the Heart
- Current Understanding of COVID-19 Pathogenesis: Cardiovascular Manifestations and Strategies

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**PLENARY SESSION – Mechnycyal Stromal Cells: Full Circle from Basic Research Insights to Clinical Trial Updates**

**Chair:** Caroline Blumer-Toti

**Speakers:**
- Stefano Balestri
- Stefano Balestri
- Chimeric Receptor NKG2D: the NK-T Cell Approach

---

**CORPORATE SYMPOSIUM HOSTED BY MILTENYI BIOTEC – Advanced Solutions for Cutting-Edge Cell Therapies**

**Chair:** Daniel J. Weiss

**Speakers:**
- Juntao Tang
- Adrian Woolfson
- Massimiliano Gnecchi
- Dominique Forge
- Loic Fieschi
- Pluripotent Stem Cells-Derived Endothelial Cells

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**PLENARY SESSION – iPSC for Cardiovascular Regenerative Medicine and Disease Modelling**

**Chair:** Daniel J. Weiss

**Speakers:**
- Massimiliano Gnecchi
- Dominique Forge
- Loic Fieschi
- Pluripotent Stem Cells-Derived Endothelial Cells

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**PLENARY SESSION – Gene Therapy Clinical Trials**

**Chair:** Sandeep Soni

**Speakers:**
- Adrian Woolfson
- Dominique Forge
- Loic Fieschi
- Pluripotent Stem Cells-Derived Endothelial Cells

---

**Preseason PLENARY – Mastering Pluripotent Cells with Therapeutic Intent**

**Chair:** John Raske

**Speakers:**
- Rudolf Janisch
- Naeimeh Rezaei
- Dominique Forge
- Pluripotent Stem Cell Therapies After the First Points of Concept: the Next Steps Forward
<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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<tbody>
<tr>
<td>09:00-12:00</td>
<td>PLENARY SESSION – BASICS AND TRANSLATIONAL POTENTIAL OF EXTRACELLULAR VESICLES INCLUDING EXOSOMES</td>
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<tr>
<td>12:00-13:30</td>
<td>LUNCH</td>
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<td>13:30-15:00</td>
<td>PLENARY SESSION – GENE ENGINEERING: THE PAST, PRESENT, AND THE FUTURE</td>
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<td>15:00-15:30</td>
<td>SESSION ENDS</td>
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**PLENARY SESSION – BASICS AND TRANSLATIONAL POTENTIAL OF EXTRACELLULAR VESICLES INCLUDING EXOSOMES**

Chair: George Mauchle

- Krakowski, A. – Mesenchymal Stromal Cell Heterogeneity: Concept and Clinical Impact
- Pekny, P. – Human Skeletal Stem/Pre-germin Cell Therapy in OA: Updates and Perspectives
- Scott Rodger, M. and Nicholas Pizzuti – The Challenges and Opportunities in Establishing Collaborative Networks and Registries for Cellular Therapy in OA

**ORAL ABSTRACT PRESENTATIONS:**

- Rola Baric – Immune Repairing of Human Subjects After Extracorporeal Mesenchymal Stromal Cell Therapy
- Philip Lee – Manufacturing Development of SENTI-105, A Gene Circuit Modified Allogeneic Bone Marrow Derived Mesenchymal Stromal Cell (BM-MSC) Therapy for the Treatment of Scleroderma
- Xiuren Zhang – Mesenchymal Stromal Cells Alleviate Experimental Acute Respiratory Distress Syndrome through the Cholinergic Anti-Inflammatory Pathway
- Camilo Cañas – Evaluating the Therapeutic Potential of Bone-Marrow Mesenchymal Stromal Cells and Exercise in a Post-Traumatic Osteoarthritis Model

**PLENARY SATELLITE SESSION – MOVING AHEAD AT FULL SPEED: IMMUNE-MEDIATED THERAPEUTICS WITH A SPOTLIGHT ON BIG PHARMA**

Chair: George Mauchle

- Marcello Atzei – Translational Immunotherapies for Cancer: Targeting Somatic Mutations
- Alberto Santagastino – The Necessary Industrialization Journey of Manufacturing in Bringing Cell and Gene Therapies to the Masses
- Mauro Castellarin – Bringing CAR-T To Patients: Delivering Sustainably on the Promise of Cell and Gene Therapies

**PLENARY SATELLITE SESSION – OXIDATIVE STRESS AS A DRIVER FOR IMMUNE-MEDIATED THERAPEUTICS**

Chair: Ricardo T. Maziarz

- Ronald J. Hause
- Chaolemeng Bao
- Gaurav Sutrave

**CORPORATE SYMPOSIUM HOSTED BY NOVARTIS – CAR-T FUNDAMENTALS: BIOLOGY, BIOMARKERS, AND KINETICS OF THE LIVING DRUG**

Chair: Richard T. Maziarz

- Use Platelet-derived Therapeutic Options for Chronic Lymphocytic Leukemia (B-LCL)
- Richard T. Maziarz – Predictors of CAR-T Cell Therapy Outcomes in B-LCL: Baseline Characteristics, Biomarkers, and Posttransfusion Factors
- Marco Manzini – Impact of Product Attributes on CAR-T Cell Therapy Outcomes

**PLENARY SESSION – GENE ENGINEERING: THE PAST, PRESENT, AND THE FUTURE**

Chair: Sandee Sam

- Donald B. Kahn – Hematopoietic Stem Cell Genomic Engineering
- Oiva Kovanen – The Use of Metabolic Engineering Strategies in Human Antigens
- Chuck O’Neil – The Pre-Clinical Development of BMN-270, an AAV Gene Therapy for Hemophilia A

**PLENARY SATELLITE SESSION – MOVING AHEAD AT FULL SPEED: IMMUNE-MEDIATED THERAPEUTICS WITH A SPOTLIGHT ON BIG PHARMA**

Chair: George Mauchle

- Ronald J. Hause – Statistical Learning Approaches for Predicting Biosimilars and Outsourced Drug Product Company From Donor-Sourced Material Composition
- Vike Narayan – Novel Chimera Receptor T Cell Therapy for Advanced Prostate Cancer

**ICST ANNUAL GENERAL MEETING & 2020 ICST CAREER ACHIEVEMENT AWARD PRESENTATION TO DR. MALCOLM BRENNER, MD, PhD**

**ICST CSD GLOBAL SHOWCASE ON COVID-19, PART II “A NEW HOPE”**

Chair: Daniel J. Weiss

- Maria Cencic – Immune-Gene Therapy Approaches – Anti-Viral Approaches and Anti-Cytokine Storm Approaches
- Daniel J. Weiss – MSC-Based Approaches: Overview of Existing Mechanisms, Pre-Clinical and Clinical Data
- Marion Khayat – MSC-Based Approaches: Review of Clinical Trials Part I
- Patricia W. Rosen – MSC-Based Approaches: Review of Clinical Trials Part II
- Anthony Ying – MSC-Based Approaches: Industry Trials

**CLOSING REMARKS**

Brigitte Lima, PhD
ACCESS DOESN’T END MAY 29


- 12 MONTHS OF UNLIMITED ACCESS TO EDUCATIONAL CONTENT:
  - ALL Recordings of LIVE and Pre-Recorded Sessions *(Subject to presenter approval)*
    - 55+ educational sessions (webcam/audio presentations with slides) Recordings of LIVE sessions will be available as of Tuesday, June 2.
  - ALL electronic posters *(Subject to presenter approval)* and the ISCT 2020 Cytotherapy Abstract Supplement

- ENDURING B2B OPPORTUNITIES WITH 12 MONTHS OF EXHIBIT HALL ACCESS
  - View product demo videos and up-to-date resources
  - Message booth representatives to continue conversations or make new connections

- PARTNERING FORUM AVAILABLE 24/7 UNTIL JUNE 4, 2020

ISCT 2020 PARIS VIRTUAL ON DEMAND
## ON DEMAND PRE-RECORDED SESSIONS

### PLENARY SATELLITE SESSION – MSC-EV TECHNICAL CONSIDERATIONS
**Chair:** Sai Kiang Lim (SG)  
**Speakers:**  
- An Hendrix (BE) – A Supporting Ecosystem to Mature Extracellular Vesicles into Clinical Application  
- Mario Gimona (AT) – Manufacturing of MSC-Derived Therapeutic Vesicular Secretome Fractions: from Preclinical Research to First-in-Human Application

### ORAL ABSTRACT PRESENTATIONS:
- Miguel Fuzeta (PT) – Scalable Production of Human Mesenchymal Stromal Cell (MSC)-Derived Extracellular Vesicles in Microcarrier-based Bioreactors under Xeno(geneic)-free Conditions *(Oral Abstract 31)*  
- Ricardo Malvicini (AR) – Bioassay Standardization to Assess Exosomes Antiinflammatory Activity In Vitro *(Oral Abstract 32)*  
- Johnatas Silva (UK) – Transfer of Mitochondria Through Msc-Derived Extracellular Vesicles Improves Alveolar-Capillary Barrier Integrity and Alleviates Mitochondrial Damage *(Oral Abstract 34)*  
- Dandan Zhu (AU) – Prematurity Negatively Impacts Therapeutic Effect of Human Amnion Epithelial Cells in Experimental Bronchopulmonary Dysplasia *(Oral Abstract 35)*

### PLENARY SATELLITE SESSION – INDUSTRIALIZING CLINICAL DELIVERY
**Chair:** Julie Murrell (US)  
**Speakers:**  
- Jacqueline Barry (UK) – How Scale Is Impacting the Ability for Clinical Sites to Manage Advanced Therapies  
- Miguel Forte (BE) – Strategic Options for Manufacturing and Supply  
- Chris Herbert (US) – Advanced Therapies Logistics within a Healthcare Provider: Challenges and Opportunities

### ISCT – CBA CORD BLOOD WORKSHOP IN PARTNERSHIP WITH WMDA AND ASTCT
**Co-Chairs:** Elizabeth J. Shpall (US) & Joanne Kurtzberg (US)  
**Speakers:**  
- Éliane Gluckman (FR) – Optimizing Results of Cord Blood Transplants  
- Katy Rezvani (US) – CAR NK Cell Therapy  
- Catherine Bollard (US) – Cord Blood Derived Virus-Specific T Cells – Broadening Applicability  
- Mayela Mendt (US) – Cord Blood Tissue Derived Exosomes for Clinical Use  
- Joanne Kurtzberg (US) – Cord Tissue MSCs, a Novel Therapeutic Cell for Immune Modulation  
- Heidi Elmoazzen (CA) – The Effect of the COVID Pandemic on Cord Blood Banking

### HOT TOPIC SESSION – REGENERATIVE MEDICINE IN DIGESTIVE DISEASES: STATE OF THE ART, OPEN NEEDS AND NEXT STEPS
**Co-Chairs:** Basak E. Uygun (US) and Giuseppe Orlando (US)  
**Speakers:**  
- Khalil N. Bitar (US) – New Approaches in Tissue Engineering and Cell Therapy in Gastrointestinal Motility  
- Vincenzo Cardinale (IT) – New Acquisitions into Liver Regenerative Medicine: From Stem Cell Niches to Clinical Applications  
- Pedro Baptista (ES) – Whole-Liver Bioengineering: The Future of Transplantation Medicine

### STATE OF THE ART: SUCCESS AND FAILURE OF CELL THERAPY CLINICAL TRIALS FOR ACUTE AND CHRONIC LUNG DISEASES
**Co-Chairs:** Patricia R M Rocco (BR) and Claudia Dos Santos (CA)  
**Speakers:**  
- Daniel J. Weiss (US) – The Global Impact of Cell Therapy for Acute and Chronic Lung Diseases  
- Shirley Mei (CA) – Cell Therapies for Lung Diseases: Building on Preclinical Evidence Towards Successful Clinical Translation  
- Anthony Ting (US) – Bone Marrow Derived Adult Stem Cells (MAPCs) for ARDS  
- Bernard Thebaud (CA) – Lessons Learned and How to Improve Future Clinical Trial Design  
- Patricia R. M. Rocco (BR) – Clinical Trials for Chronic Lung Diseases  
- John Laffey (IE) – Future Perspectives: Proposal for the Creation of an International Stem Cell Clinical Trial Network for Lung Diseases
ON DEMAND PRE-RECORDED SESSIONS

90 min

SAFETY MATTERS AND KEY CONSIDERATIONS FOR REGENERATIVE MEDICINE PRODUCTS – JOINT SESSION WITH EBMT AND TERMIS EU

Co-Chairs: Ivan Martin (CH) and Joan Garcia (ES)
Speakers:
- Fermín Sánchez-Guijo (ES) – POC Manufacturing of MSC
- Ineke Slaper-Cortenbach (NL) – Safety of Regenerative Tissue Products, Traceability and Other Safety Issues
- Marina Maréchal (BE) – Introduction to the RM Terminology
- Julie Allickson (US) – Examples of ISBT 128 Coding and Labeling of RM Products and Future Needs
- Christian Chabannon (FR) – The Need for Registries and Real-World Data to Fully Assess the Medical Value of Cell and Gene Therapies

IMAGING CELLULAR THERAPEUTICS: JOINT SESSION WITH HESI CT-TRACS

Chair: Brooke Helfer (US)
Speakers:
- Jane Sosabowski (UK) – Developments in Imaging Cell-Based Therapy: Applications in Cancer Immunotherapies
- Bettina Weigelin (DE) – Seeing is Believing – In Vivo Microscopy for Optimizing Cellular Immunotherapies
- Vidya Gopalakrishnan (US) – Monitoring Intracerebellar Delivery of Natural Killer Cells

GENE THERAPY FOR GLOBIN DISORDERS

Chair: Sandeep Soni (US)
Speakers:
- Marina Cavazzana (FR) – Gene Therapy for β-Hemoglobinopathies
- Rahul Palchaudhuri (US) – A Single Dose of CD117 Antibody Drug Conjugate Enable Hematopoietic Stem Cell Based Gene Therapy in Nonhuman Primates
- Sandeep Soni (US) – Gene-Editing for Hemoglobinopathies

90 min

ORAL ABSTRACT SHOWCASE

Chair: Daniel J. Weiss (US)

ORAL ABSTRACT PRESENTERS:

- Esmond Lee (US) – Gene Editing using CRISPR Enables FOXP3 Gene Repair in HSPCs and IPEX patient T Cells (Abstract O22)
- Massimiliano Paganelli (CA) – Safe and Effective Treatment of Acute Liver Failure by Allogeneic Transplantation of Stem Cell-Derived Encapsulated Liver Tissue without Immunosuppression (Abstract O13)
- Glaucio Souza (US) – Magnetic 3D Bioprinting for Personalized Medicine (Abstract O25)
- Jun Xu (US) – Predictive Modeling Demonstrating a Two-Factor Signature as Early as a Week Prior to Harvest for Chimeric Antigen Receptor (CAR) T-Cell Manufacturing Growth (Abstract O16)
- Mason Chilmonczyk (US) – Probing MSC and Tumor Cell Secretome Locally via Dynamic Sampling Platform (DSP) (Abstract O10)
- Raniero Chimienti (IT) – Engineering of NK Activating Receptor Ligands Enhances Immune Compatibility of MHC-I/- iPSC-Derived β Cells for Cell Therapy of Type 1 Diabetes (Abstract O11)
- Jae Young Lee (KR) – Enhancing the Therapeutic Potential of Mesenchymal Stem Cell-Based Therapy via CRISPR/Cas9-Based Genome Editing (Abstract O12)
- Rui Tostoes (US) – Acoustic Affinity Cell Selection: a Non-Paramagnetic Scalable Technology for T Cell Selection from Unprocessed Apheresis Products (Abstract O14)
- Jack Hayes (US) – Mesenchymal Stem Cell Therapy Improves Pulmonary Function and Exercise Tolerance in Patients with Chronic Obstructive Pulmonary Disease (COPD) and High Baseline Inflammation (Abstract O16)
- Dror Ben-David (IL) – Bonofill-II, from Bench to Bedside: a Novel Autologous Cell-Based, Tissue-Engineered Product in Line to Replace Bone Autografts for Large Segmental Bone Defect Applications (Abstract O23)
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<tr>
<th>Session Title</th>
<th>Duration</th>
<th>Description</th>
<th>Chairs</th>
<th>Speakers</th>
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<tbody>
<tr>
<td>ISCT Presidential Task Force Session – Emerging Issues in Unproven Cell Therapies</td>
<td>90 min</td>
<td>Co-Chairs: Aaron Levine (US) and Laertis Ikonomou (US)</td>
<td>Aaron Levine (US) – Ethical and Policy Considerations Associated with Speculative Cell Banking Services, Eva Rohde (AT) – Extracellular Vesicle-Based Unproven Therapies, Patti Zettler (US) – Situating Stem Cells within the Regulatory Environment for Patient-Driven Access, Massimo Dominici (IT) &amp; Laertis Ikonomou (US) – ISCT Presidential Task Force on the Use of Unproven and Unethical Cell &amp; Gene Therapies</td>
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<tr>
<td>Strategies for Commercialization Track Session – Innovating Process Development for Sustainable Manufacturing</td>
<td>90 min</td>
<td>Chair: Dominic Clarke (US)</td>
<td>Stuart Curbishley (UK) – Challenges in Translating Academic and Start-Up Companies to First in Man and Early Phase Clinical Trials, Jean-François Chaubard (BE) – Allogeneic T-Cell Therapies: Shifting Toward Commercial Manufacturing, David Smith (US) – Survival Guide to CDMO</td>
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<tr>
<td>Strategies for Commercialization Track Session – Can We Industrialize CAR T Therapies? The Practical Challenges in Achieving Wide-Spread Patient Access</td>
<td>90 min</td>
<td>Chair: William Milligan (CA/TW)</td>
<td>Gunther Busam (CH) – How to Commercialize Autologous CAR-Ts, Elizabeth Hexner (US) – Industrializing CAR Ts: What Can We Learn From 50+ Years of Stem Cell Transplantation?, Suma Rao (US) – Development Challenges When Your Raw Material is Human and the Product is a Population, Wen Bo Wang (US) – Developing Allogeneic Cancer Immunotherapy with iPSC Technology</td>
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<tr>
<td>Strategies for Commercialization Track Session – Clinical Experience in Regenerative Medicine and Tissue Engineering, The Next Wave of Advanced Therapies</td>
<td>90 min</td>
<td>Chair: Julie Allickson (US)</td>
<td>Julie Allickson (US) – Clinical Translation of Tissue Engineering in an Academic Facility, Petter Björquist (SE) – Personalized Tissue-Engineered Organs that Will Revolutionize Future Medicine, Laura Niklason (US) – Will Engineered Tissues Transform Medicine?</td>
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## ON DEMAND PRE-RECORDED SESSIONS

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<th>Duration</th>
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<th>Chair(s)</th>
<th>Speakers</th>
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</table>
| 90 min   | QUALITY AND OPERATIONS TRACK SESSION – MANUFACTURING PROCESS VALIDATION – CONSIDERATIONS FOR AUTOLOGOUS PRODUCTS | Emily Hopewell (US) | Antonio Ruiz-Garcia (ES) – Validation of the Process for Autologous vs Allogeneic Products and Tissue Products  
Corey Smith (AU) – Impact of the Use of Healthy vs Diseased Donors on the Development of T Cell Therapies  
Aisha Khan (US) – Examples of Process Validation of the Autologous vs Allogeneic: Schwann Cells vs Mesenchymal Stem Cells Derived Exosomes |
| 90 min   | QUALITY AND OPERATIONS TRACK SESSION – CHARACTERIZATION OF ACTIVE SUBSTANCE AND COMPARABILITY | Rosemarie Bell (AU) | Mehrshid Alai-Safar (US) – Approaches to Comparability Studies of CAR-T Products  
Mo Heidaran (US) – Essential Elements of Best Practices to Follow for Demonstrating Product Comparability  
Krishnendu Roy (US) – The Role of Big-Data Analytics in Developing Predictive CQAs and CPPs, and Enable Real-Time Process Monitoring, for Cell Therapy Manufacturing |
| 75 min   | HOT TOPIC SESSION – GENE EDITED AND ENGINEERED CELL BASED THERAPEUTICS FOR CANCER | Khalid Shah (US) and Massimo Dominici (IT) | Massimo Dominici (IT) – Engineering MSC Against Tumors  
Khalid Shah (US) – Gene Edited and Engineered Edited Cell Therapies for Solid Tumors |
| 60 min   | ISCT GLOBAL REGULATORY PERSPECTIVES (GRP) WORKSHOP – PANEL DISCUSSION ON COMBINATION PRODUCTS AND STERILITY CONTROLS | |  
**KEY TOPICS IN COMBINATION PRODUCTS**  
Interviewers: Karen Nichols (US), David DiGiusto (US)  
Panelists: Melanie Eacho (US), Colin White (US), Patrick Bedford (CA)  
**STERILITY CONTROLS – PAST, PRESENT, FUTURE**  
Interviewer: Dominic Wall (AU)  
Panelists: Donald Singer (US), Arnaud Paris (FR)  
**ISCT GLOBAL REGULATORY PERSPECTIVES (GRP) WORKSHOP – REGULATORY ROUND-UP FROM NORTH AMERICA, EUROPE, AND ASIA-PACIFIC**  
Presenters:  
Scott Burger (US) – Regulatory Round-Up from North America  
Christopher Bravery (UK) – Regulatory Round-Up from Europe  
Janet Macpherson (AU) – Regulatory Round-Up from Asia Pacific |
LIVE SESSIONS
ALL LIVE SESSIONS ARE IN THE PLENARY HALL

What time is it for me?  Bookmark session in my calendar

ISCT CSO Global Showcase on COVID-19, PART 1 “The Force Awakens”
Starts at 14:30 CEST • Plenary Hall

Chair: Daniel J. Weiss (US)
In the first of two sessions centered on the COVID-19 pandemic, focus will be on pathogenesis and on experiences of health care workers on the front lines in Italy, New York City, and China. ISCT members have been at the forefront of both clinical care, clinical and mechanistic investigations, and the impact of travel and shipping restrictions on clinical cell therapy programs. An interactive discussion will follow presentations from an outstanding panel of global speakers.

CURRENT UNDERSTANDING OF COVID-19 PATHOGENESIS: CYTOKINE STORM AND RESPIRATORY PATHOLOGY
Mauro Krampera (IT)

CURRENT UNDERSTANDING OF COVID-19 PATHOGENESIS: CARDIOVASCULAR MANIFESTATIONS AND STRATEGIES
Massimiliano Gnegchi (IT)

REPORTS FROM THE FRONT LINES – ITALY
Enrico Clini (IT)

REPORTS FROM THE FRONT LINES – NEW YORK, USA
Tobias Hohl (US) and Santosh Vardhana (US)

COVID-19 INFECTION: THE PERSPECTIVES ON IMMUNE RESPONSES
Yufang Shi (CN)

SUPPLY CHAIN ISSUES – NORTH AMERICA
Diane Kadidlo (US)

GLOBAL SUPPLY CHAIN ISSUES
Heidi Elmoazzen (CA)

Plenary Session – Mesenchymal Stromal Cells: Full Circle from Basic Research Insights to Clinical Trial Updates
Starts at 16:30 CEST • Plenary Hall

Supported by an Unrestricted Educational Grant from Takeda

Chair: Sowmya Viswanathan (CA)
This session will bring together speakers who will provide a full spectrum overview of latest developments in the field of mesenchymal stromal cell research. This includes insights into newly hypothesized mechanisms of action and single cell characterization of mesenchymal stromal cells, clinical translation challenges using pooled bone marrow-derived mesenchymal stromal cells for treatment of refractory Graft-vs. Host Disease, and updates from a pioneering European trial on the use of mesenchymal stromal cells to treat refractory Sclerodoma.

ENGINEERING A HAEMATOPOIETIC STEM CELL NICHE BY REVITALIZING MESENCHYMAL STROMAL CELLS
Paul Frenette (US)

A UNIFYING THEORY FOR MSC THERAPEUTIC POTENCY IN MITIGATION OF TISSUE INJURY SYNDROMES
Jacques Galipeau (US)

Focusing on evolving thinking on new discoveries regarding MSC endogenous single cell biology, exosomes, matrix functionalities, potency measurement and variables affecting potency (especially in the immune modulation space), we propose that an overlooked and potentially disruptive perspective is the impact of in vivo persistence on potency which is not predicted by the surrogate cellular functional assays performed in vitro and how this translates to in vivo outcomes. We propose a theory that MSC Potency (P) writ large is proportional to in vivo persistence (p) of
said exogenous pharmaceutical MSC: \( \text{P } \alpha \text{p} \). We further develop this theory in the following: that persistence (\( p \)) is the product of 3 variables (in ranked order): route of delivery (\( r \)), functional fitness (\( f \)), and dose (\( d \)). Therefore, (a) \( \text{P } \alpha \text{p} \) and (b) \( p \alpha r \cdot f \cdot d \).

**CHILDREN AND ADULTS WITH REFRACTORY ACUTE GRAFT-VERSUS-HOST DISEASE RESPOND TO TREATMENT WITH THE MESENCHYMAL STROMAL CELL PREPARATION “MSC-FFM” – OUTCOME REPORT OF 92 PATIENTS**

*Halvard Bönig (DE)*

Using a proprietary pooling method, Bader, Kuci, and the presenting author are generating off-the-shelf cryopreserved MSCs of pharmaceutical quality – with dose-to-dose equipotency and a highly robust, GMP-compliant manufacturing process. Preliminary evidence after treatment of 92 children, adolescents and adults indicates excellent tolerability and suggests efficacy. Responses were better if treatment was early, but remained clinically meaningful even at later time points. Survival after 6 months was similar for adults and children, seemed superior to survival of similar patients treated with any of the alternative medicines. A multicentric international randomized trial against best available treatment is expected to begin recruitment in Q2/2020 with the goal of obtaining a marketing authorization in Europe.

**PRE-CLINICAL BACKGROUND AND TREATMENT OF REFRACTORY SEVERE SYSTEMIC SCLEROSIS PATIENTS BY MESENCHYMAL STEM CELLS**

*Dominique Farge (FR)*

Systemic sclerosis (SSc) is an autoimmune disease with high morbidity and mortality. SSc treatments are only palliative, except autologous hematopoietic stem cell transplantation which indication remains limited to selected patients. Mesenchymal stromal cells (MSC) modulate key mechanisms driving SSc (i.e. endothelial cell damage, immune activation, inflammation and fibrosis).
Plenary Satellite Session – iPSC for Cardiovascular Regenerative Medicine & Disease Modelling

Starts at 20:15 CEST • Plenary Hall

Supported in part by BlueRock Therapeutics

Chair: Daniel J. Weiss (US)

Organized by the ISCT Cardiovascular Scientific Subcommittee, this session will shine a spotlight on pluripotent stem cells as a promising therapeutic approach to cardiovascular diseases and highlight both pre-clinical and clinical research currently being undertaken to drive the translation of iPSC-based regenerative medicine. The session will conclude by featuring a series of top scoring abstract submissions addressing emerging research in iPSC followed by an interactive Q&A with keynote presenters.

INDUCED PLURIPOTENT STEM CELLS FOR CARDIOVASCULAR DISEASES MODELLING AND DISCOVERY OF PERSONALIZED THERAPIES

Massimiliano Gnecchi (IT)

USE OF PLURIPOTENT STEM CELLS FOR REGENERATING/REPAIRING THE HEART

Philippe Menasché (FR)

The talk will address the rationale for using PSC and more specifically their cardiac-committed derivatives for repairing the heart, their current use in the clinics and the associated technical challenges and the perspective of leveraging their paracrine properties to exclusively deliver their secretome.

ORAL ABSTRACT PRESENTATIONS:

BUILDING FUNCTIONAL VASCULAR GRAFTS FROM HUMAN PLURIPOTENT STEM CELLS-DERIVED ENDOTHELIAL CELLS (Oral Abstract 1)

Gabor Foldes (UK)

HUMAN VASCULARISED MESENCHYMAL SPHEROIDS HIGHLIGHT THE ROLE OF WDR35 IN BONE FORMATION (Oral Abstract 2)

Loic Fievet (FR)

HUMAN KIDNEY ORGANOIDS PRODUCE FUNCTIONAL RENIN (Oral Abstract 3)

Anusha Shankar (NL)

Presidential Plenary Session – Mastering Pluripotent Cells with Therapeutic Intent

Starts at 22:00 CEST • Plenary Hall

Chair: John Rasko (AU)

Unlocking the therapeutic potential of embryonic and induced pluripotent cells, this session will offer novel insights into the use of pluripotent cells in the treatment of neurological and developmental disorders as well as highlight recent advancements towards clinical application of hPSCs for monogenic diseases.

EPIGENETIC REGULATION IN DEVELOPMENT AND DISEASE

Rudolf Jaenisch (US)

The development of the iPS cell technology has revolutionized our ability to study development and diseases in defined in vitro cell culture systems. The talk will focus on the use of gene editing for the study of epigenetic regulation in development and disease.

1. Editing DNA methylation in the mammalian genome: The functional significance of specific methylation events in development and disease remains elusive due to lack of experimental approaches to edit these events. We developed a DNA methylation editing toolbox that fusion of either the catalytic domain of Tet1 or Dnmt3a protein to a catalytic inactive Cas9 (dCas9) to achieve targeted DNA methylation editing with co-expression of target-specific guide RNAs. Our results established that a modified CRISPR system with dCas9 fused by DNA modification enzymes can be assembled into DNA methylation editing tools to study the functional significance of specific methylation event in the mammalian genome. Finally, we show that these tools can edit DNA methylation in mice, demonstrating their wide utility for functional studies of epigenetic regulation.

2. Epigenetic regulation and disease: reversal of gene silencing in Fragile X and Rett Syndrome: The
expansion of a GGC repeat leads to methylation and silencing of the FMR1 gene, which is the cause for Fragile X Syndrome. We have used the epigenetic gene editing tools to induce hypomethylation the repeat expansion leading to expression of the FMR1 gene and reversal of the disease specific cellular phenotype. In Rett syndrome, the mutation of the X-linked MECP2 gene is causative for the disease. I will discuss our efforts to activate the wt MECP2 allele carried on the inactive X chromosome.

**ENGINEERING FATE AND FUNCTION FROM PLURIPOTENT STEM CELLS**

Pete Zandstra (CA)

**PLURIPOTENT STEM CELL THERAPIES AFTER THE FIRST PROOFS OF CONCEPT: THE NEXT STEPS FORWARD**

Marc Peschanski (FR)

Derivatives of pluripotent stem cell lines are now tried clinically for regenerative medicine in various pathological indications. I will discuss the scientific bases on which those clinical applications are built and describe their process taken the example of our ongoing trial for retinitis pigmentosa. I will then identify main areas of progress and envision current limitations of those therapies.

**Plenary Satellite – Gene Therapy Clinical Trials**

Chair: Sandeep Soni (US)

**PRECISION ENGINEERING OF THE HUMAN GENOME**

Adrian Woolfson (US)

**EX-VIVO MANIPULATION OF HEMATOPOIETIC STEM CELLS FOR HEMOGLOBINOPATHIES**

Sandeep Soni (US)

This talk will provide an update on the current status of clinical trials for ex-vivo genetic engineering of human HSPCs for thalassemia and sickle cell disease.

**ORAL ABSTRACT PRESENTATIONS:**

**THE GENETICALLY-ENGINEERED STEM CELL THERAPY OF HUNTINGTON DISEASE: SPT4 KNOCKOUT HD PATIENT IPSC-NPCS TRANSPLANTATION RESCUE ABNORMAL NEURONAL** (Oral Abstract 29)

HyunJung Park (KR)

**CONSTRUCTION OF A NOVEL, DUAL, SELF-REPLICATING MINICIRCLE; AN EFFICIENT TOOL IN TRANSDIFFERENTIATION** (Oral Abstract 30)

Naeimeh Rezaei (IR)

**Plenary Session – Basics and Translational Potential of Extracellular Vesicles Including Exosomes**

Starts at 13:00 CEST • Plenary Hall

Chair: Bernd Giebel (DE)

Most cells release Extracellular Vesicles (EVs) into their environment. In particular, small EVs of 50-200 nm that include exosomes, have been shown to mediate intercellular communication in many physiological and pathophysiological processes. Small EVs from some cell sources, e.g. mesenchymal stromal cells (MSCs), can alleviate pathological processes and are considered as novel therapeutic agents. Within the session basic aspects of EV biology and their therapeutic potential will be discussed.

**EXOSOMES AND OTHER EXTRACELLULAR VESICLES: UNRAVELING COMMON AND DIFFERENT PROPERTIES FOR THERAPEUTIC USE**

Clotilde Théry (FR)

Basic aspects of EV biology will be provided and methods to prepare and characterize EVs will be discussed, including the criteria provided by the International Society of EVs (ISEV) as Minimal Information for Studies of EVs 2018 (MISEV2018).
THERAPEUTIC POTENTIAL OF MSC-SEVS: PROMISES AND CHALLENGES

Sai Kiang Lim (SG)

The promising therapeutic potential of MSC-derived small EVs (MSC-sEVs) to treat intractable diseases will be discussed together with the challenges in developing MSC-sEVs into safe, potent therapeutics including requirements to describe their identity and potency.

AMNIOTIC EPITHELIAL CELL DERIVED EVS FOR BRONCHOPULMONARY DYSPLASIA: CONSIDERATIONS FOR NEONATAL INDICATIONS

Rebecca Lim (AU)

Dr. Lim will be discussing her lab’s observations on how different donor characteristics such as prematurity, twins vs singletons, gestational diabetes etc influence EV properties and how these have to be considered when evaluating their therapeutic potential.

Plenary Satellite – From Mechanism of Action to Establishing Collaborative Networks: The Opportunities, Challenges and Tangible Progress in the Development of Cell-Based Therapies for Musculoskeletal Disease

Chair: George Muschler (US)

This session will analyze the building blocks, opportunities and obstacles faced in the formation of collaborative networks to advance the field of cell-based therapeutics for musculoskeletal disease. The session will begin by addressing the clinical impact of cell heterogeneity and plasticity followed by an overview of European consortia addressing standardized protocols for tissue specific progenitor cell fabrication. The current collaborations in place for clinical trials will also be reviewed. Furthermore, the importance of establishing collaborative networks to investigate, define, and translate tissue specific progenitor cell-based therapeutics for musculoskeletal application will be addressed.

MESENCHYMAL STROMAL CELL HETEROGENEITY: CONCEPT AND CLINICAL IMPACT

Karin Tarte (FR)

Dr. Karin Tarte will discuss the heterogeneity and plasticity of human mesenchymal stromal cells (MSC) and how it could impact their clinical use. In particular, she will present recent data on the influence of tissue origin, donor characteristics, and culture conditions on the functional features of clinical-grade MSC. Such data paves the way for a better understanding of the critical parameters that could influence the choice of MSC for therapeutic applications.

HUMAN SKELETAL STEM/PROGENITOR CELL THERAPY IN OA: UPDATE AND PERSPECTIVES

Christian Jorgensen (FR)

THE CHALLENGES AND OPPORTUNITIES IN ESTABLISHING COLLABORATIVE NETWORKS AND REGISTRIES FOR CELLULAR THERAPY IN OA

Scott Rodeo (US) and Nicolas Piuzzi (US)

Progress in the area of cell therapy for treatment of osteoarthritis of the knee will require:
1. Development of registries and collection of standard patient-reported outcome measures (PROMs)
2. Robust methods to characterize OA
3. Biospecimen repositories

There are human, financial, and technology-related challenges in establishing registries. We need to leverage technology to develop digital solutions to ease patient burden. The process may be facilitated by making data collection “standard of care”. We need to identify standardized and validated Patient Reported Outcomes Measures (PROMs) that can be used across different institutions. A registry needs to include robust and comprehensive data on patient demographics and underlying pathology, along with detailed documentation of the state and severity of OA, given the very heterogeneous nature of OA. In addition to the collection of clinical and demographic data...
data, we need to identify and quantify the composition and biologic activity of various cell therapy formulations used in clinical trials, so that we can ultimately correlate the clinical and imaging outcomes with the specific cell treatment received by the patient. This process will be aided by defining sentinel markers of quality, potency, purity, etc. for different conditions and pathologies. It is critically important to identify specific tests that can be performed on cell therapy formulations that are logistically and financially reasonable and that are validated to document the quality, potency, and biologic activity of these treatments.

**ORAL ABSTRACT PRESENTATIONS:**

**IMMUNE REPROGRAMMING IN HUMAN SUBJECTS AFTER EXTRACORPOREAL MESENCHYMAL STROMAL CELL THERAPY** (Oral Abstract 5)  
*Rita Barcia* (US)

**MANUFACTURING DEVELOPMENT OF SENTI-101, A GENE CIRCUIT MODIFIED ALLOGENEIC BONE MARROW DERIVED MESENCHYMAL STROMAL CELL (BM-MSC) THERAPY FOR THE TREATMENT OF SOLID TUMORS** (Oral Abstract 6)  
*Philip Lee* (US)

**MESENCHYMAL STROMAL CELLS ALLEVIATES EXPERIMENTAL ACUTE RESPIRATORY DISTRESS SYNDROME THROUGH THE CHOLINERGIC ANTI-INFLAMMATORY PATHWAY** (Oral Abstract 7)  
*Xiaoran Zhang* (CN)

**EVALUATING THE THERAPEUTIC POTENTIAL OF BONE-MARROW MESENCHYMAL STROMAL CELLS AND EXERCISE IN A POST-TRAUMATIC OSTEOARTHRITIS MODEL: FUNCTIONAL AND RADIOGRAPHIC ANALYSIS** (Oral Abstract 8)  
*Camila Carballo* (US)

**ENGINEERED NASAL CARTILAGE FOR THE REPAIR OF OSTEOARTHRITIC KNEE CARTilage DEFECTS** (Oral Abstract 9)  
*Karoliina Pelttari* (CH)

**Plenary Session – Is Big Pharma Ready for Industrial Scale Cell and Gene Therapy?**  
*Chair: Anthony Ting* (US)

Recent successes in the market authorization of cell and gene therapy products has generated tremendous excitement. However, to be successful, the industry now needs to move into a phase of industrialization, where like the car industry it needs the “eureka” moment of standardization that allows the efficient flow from product to patient. To realize this, we need to overcome the barriers between all the silos in the system (e.g. hospital, manufacture, testing, etc.) so that patients are efficiently treated.

**TOWARDS INDIVIDUALIZED CELL THERAPY IN CANCER: TARGETING SOMATIC MUTATIONS**  
*Angela Krackhardt* (DE)

Cellular therapy has shown high efficacy in the treatment of hematological malignancies by the use of T cells genetically modified by chimeric antibody receptors (CAR). Two CAR T cell products have been approved so far and are applied to patients in defined clinical indications in an industrial scale. However, although many novel approaches are currently followed, this approach has demonstrated less efficacy in the treatment of solid malignancies so far, especially due to limits in defining suitable surface target antigens. Nevertheless, highly suitable target antigens represented by mutated peptide ligands derived from somatic mutations are presented by major histocompatibility complexes (MHC) in most individual cancers which may be identified by mass spectrometry-based approaches. Such epitopes may be recognized by high avidity T cell receptors (TCR) derived from the autologous or allogeneic repertoire. Neoantigen-specific TCR and derivates therefore represent highly attractive tools for genetic modification of non-dysfunctional T cells. The development of cellular therapies using neoantigen-specific TCR-modified effector cells represents a highly attractive therapeutic approach facing, however, major hurdles with respect to regulatory and industrial
challenges of clinical translation. Finding solutions for these challenges will be of major importance to provide such therapies to a larger patient population in future.

THE NECESSARY INDUSTRIALIZATION JOURNEY OF MANUFACTURING IN BRINGING CELL AND GENE THERAPIES TO THE MASSES

Alberto Santagastino (CH)

Learning objectives:
• As the industry moves from clinical to commercial, we are reaching an inflection point and face key challenges.
• Manufacturing is the focus and is under critical pressure from a technical, timeline and cost perspective.
• Process development will be key to industrializing the manufacturing and achieving commercial viability.
• CDMOs have a key role to play in delivering industrialization, technologies, capacity and expertise to the industry

BRINGING CAR-T TO PATIENTS: DELIVERING SUSTAINABLY ON THE PROMISE OF CELL AND GENE THERAPIES

Emanuele Ostuni (CH)

Learning objectives:
• Basics of CAR T science
• CAR T Supply chain and its requirements
• CAR T business model and approaches
• Basics of value-based healthcare
• Complexity of reimbursement
• Future of CAR T

Plenary Session - Gene Engineering: The Past, Present and The Future

Starts at 21:00 CEST • Plenary Hall

Chair: Sandeep Soni (US)

Gene-engineering is currently ‘in-vogue’ as a potential treatment for multiple monogenic and rare diseases. This session will highlight the progress in the field of gene-insertion, vector biology and gene-editing for both the ex-vivo and in-vivo approaches of gene manipulation. The current challenges and safety issues will be discussed, with the spotlight on ongoing clinical trials as examples of potential curative therapies.

HEMATOPOIETIC STEM CELL GENE THERAPY

Donald B. Kohn (US)

Dr. Kohn will review clinical and pre-clinical studies on gene modification of autologous hematopoietic stem cells to treat genetic blood cell diseases.

THERAPEUTIC GENE EDITING STRATEGIES IN FANCONI ANEMIA

Paula Rio (ES)

Fanconi anemia is a rare disease characterized by congenital abnormalities, increased cancer predisposition and early bone marrow failure. Recent results from our laboratory have shown that corrected cells using lentiviral vectors can engraft in the patients in the absence of any conditioning, eliminating potential side effects associated to allogeneic bone marrow transplantation.

In this context, gene editing has emerged as a potential strategy to accurately correct specific disease related mutations. Since non-homologous end-joining (NHEJ) is the preferential DNA-repair mechanism in HSCs, particularly in the case of FA cells, we aimed at exploiting this pathway for the compensation of FA associated mutations, mimicking the spontaneous reversions observed in mosaic patients.

Previous experiments conducted in lymphoblastic cell lines (LCLs) showed the ability of NHEJ to correct two different mutations frequently found in FA-A patients. A similar gene editing approach was then used to correct primary CD34+ cells from FA-A patients. Remarkably, NHEJ-mediated editing induced an \textit{in vitro} proliferative advantage in these cells, as previously shown with lentiviral vectors and also corrected their characteristic FA-cell phenotype. In contrast to HDR, we found that the efficacy of NHEJ to edit primitive human HSCs was comparable to that observed in more mature progenitor cells, indicating that NHEJ-editing approaches should constitute a good approach for the editing of long-term repopulating HSCs.
Moving towards the clinical application of NHEJ-mediated repair we focused on improving gene editing efficiency in HSCs and expanding its applicability to other FA complementation groups. Using chemically modified small guide RNAs (MS-sgRNAs) editing efficacy reached levels of 89% in healthy donor hematopoietic stem/progenitor cells.

To confirm the broader applicability and the efficiency of this approach in other FA complementation groups we selected FA lymphoblastic cells lines (LCLs) harboring mutations in FANCB, FANCC, FANCD1 (BRCA2) and FANCD2. The NHEJ-mediated editing was robustly efficient reaching an 80% editing events with an average of potentially therapeutic indels ranging from 20 to 30%, being particularly remarkable in the FA-D1 subtype, characterized by a marked defect in homologous directed repair (HDR).

All together these results demonstrate that the CRISPR/Cas9 induced NHEJ-mediated editing constitutes a simple and efficient strategy that could be applied for the treatment of specific FA mutations in all complementation groups.

THE PRE-CLINICAL DEVELOPMENT OF BMN 270, AN AAV5 GENE THERAPY FOR HEMOPHILIA A

Charles A. O’Neill (US)

Data will be presented to address key questions in the pre-clinical development of an AAV-based gene therapy

- Proof-of-concept studies demonstrating efficacy in a mouse model of hemophilia A
- Transgene DNA biodistribution in mice
- Changes in the expression profile as a function of age in mice
- Evaluation of germline transmission risk

Plenary Satellite – Moving ahead at full speed: Immune-Mediated Therapeutics with a Spotlight on Big Data Analyses and Solid Tumors

Chair: Bruce Levine (US)

STATISTICAL LEARNING APPROACHES FOR PREDICTING LISOCABTAGENE MARALEUCEL (LISO-CEL; JCAR017) DRUG PRODUCT COMPOSITION FROM DONOR-SELECTED MATERIAL COMPOSITION

Ronald J. Hause (US)

Lisocabtagene maraleucel (liso-cel; JCAR017) is an investigational, CD19-directed, defined composition, 4-1BB CAR T cell product administered at equal target doses of CD8+ and CD4+ CAR+ T cells.

The liso-cel manufacturing process is designed to minimize variability in cellular composition introduced by the leukapheresis starting material.

To improve our understanding of the effects of donor selected T cell material (SMAT) variability on liso-cel drug product (DP) quality, we developed a statistical approach that leverages canonical correlation analysis (CCA) and lasso regression to better understand and predict CAR T cell composition.

NOVEL CHIMERIC ANTIGEN RECEPTOR T CELL THERAPY FOR ADVANCED PROSTATE CANCER

Vivek Narayan (US)

Learning Objectives:

- Describe the challenges for CAR-T therapies for advanced solid tumors.
- Review the rationale and early experience with prostate specific membrane antigen (PSMA) as a target antigen for CAR-T therapy for advanced prostate cancer.
ISCT CSO GLOBAL SHOWCASE ON COVID-19, PART II ‘A New Hope’

This second COVID-19 session will focus on novel cell-based and immunologic-based therapeutics. With the ongoing threat of emerging infectious disease, what role can cell therapies play therapeutically and how should studies be conducted and interpreted? Through translational and clinical science, a new hope for the development of novel therapeutics arises. An interactive discussion will follow presentations from an outstanding panel of global speakers.

IMMUNO-Gene THERAPY APPROACHES – ANTI-VIRAL APPROACHES AND ANTI-CYTOKINE STORM APPROACHES

Maria Cancio (US)

MSC-BASED APPROACHES - OVERVIEW OF EXISTING MECHANISTIC, PRE-CLINICAL AND CLINICAL DATA

Daniel J. Weiss (US)

MSC-BASED APPROACHES – REVIEW OF CLINICAL TRIALS PART I

Maroun Khoury (CL)

MSC-BASED APPROACHES – REVIEW OF CLINICAL TRIALS PART II

Patricia R. M. Rocco (BR)

MSC-BASED APPROACHES – INDUSTRY TRIALS

Anthony Ting (US)
ON DEMAND PRE-RECORDED SESSIONS
ALL PRE-RECORDED SESSIONS ARE AVAILABLE IN THE BREAKOUT ROOM

Plenary Satellite – MSC-EV Technical Considerations

Chair: Sai Kiang Lim (FR)

A SUPPORTING ECOSYSTEM TO MATURE EXTRACELLULAR VESICLES INTO CLINICAL APPLICATION
An Hendrix (BE)

Learning objectives:
• Introduction to the advantages of extracellular vesicles as smart nanoscale therapeutics
• Understanding the challenges in large scale and reproducible production of extracellular vesicles-based therapeutics
• Highlighting the EV-TRACK knowledgebase to increase transparency and recombinant extracellular vesicles as biological reference material to monitor production

MANUFACTURING OF MSC-DERIVED THERAPEUTIC VESICULAR SECRETOME FRACTIONS: FROM PRECLINICAL RESEARCH TO FIRST-IN-HUMAN APPLICATION
Marion Gimona (AT)

The presentation adresses the following topics:
• Importance of GMP compliance and scalability of the manufacturing train
• Relevance of target indication for the design of clinical trial protocols
• Complexity of quality control for EVs
• Requirement for indication-specific activity and potency assays
• Data from a first-in-human application in a cochlea implantation setting

O R A L  A B S T R A C T  P R E S E N T A T I O N S:

SCALABLE PRODUCTION OF HUMAN MESENCHYMAL STROMAL CELL (MSC)-DERIVED EXTRACELLULAR VESICLES IN MICROCARRIER-BASED BIOREACTORS UNDER XENO(GENEIC)-FREE CONDITIONS (Oral Abstract 31)
Miguel Fuzeta (PT)

BIOASSAY STANDARDIZATION TO ASSESS EXOSOMES ANTIINFLAMMATORY ACTIVITY IN VITRO (Oral Abstract 32)
Ricardo Malvicini (AR)

THERAPEUTIC EFFECTS OF EXTRACELLULAR VESICLES OBTAINED FROM BONE MARROW-DERIVED, ADIPOSE TISSUE-DERIVED, AND LUNG-DERIVED MESENCHYMAL STROMAL CELLS ON THE LUNG AND DISTAL ORGANS IN EXPERIMENTAL SEPSIS (Oral Abstract 33)
Natalia Blanco (BR)

TRANSFER OF MITOCHONDRIA THROUGH MSC-DERIVED EXTRACELLULAR VESICLES IMPROVES ALVEOLAR-CAPILLARY BARRIER INTEGRITY AND ALLEVIATE MITOCHONDRIAL (Oral Abstract 34)
Johnatas Silva (UK)

PREMATURITY NEGATIVELY IMPACTS THERAPEUTIC EFFECT OF HUMAN AMNION EPITHELIAL CELLS IN EXPERIMENTAL BRONCHOPULMONARY DYSPLASIA (Oral Abstract 35)
Dandan Zhu (AU)

Plenary Satellite – Industrializing Clinical Delivery

Chair: Julie Murrell (US)

Life changing therapies are being created and manufacturing systems are constantly evolving. However there also needs to be innovation within the logistical delivery of advanced therapies within the clinical setting. The supply chain is working now but how will it cope when there are tens of therapies and thousands of patients, globally? The “hidden challenge” is the last 100m within the clinical setting.

This session will highlight some key challenges that
developers need to be aware of, and present solutions that are currently being developed. Moreover, presenters will describe ways the infrastructure, equipment, staffing, training, and systems need to be designed to operate efficiently to treat the patients.

**HOW SCALE IS IMPACTING THE ABILITY FOR CLINICAL SITES TO MANAGE ADVANCED THERAPIES**

*Jacqueline Barry (UK)*

Significant advances in stem cell biology, immunology and genetic engineering have underpinned the emergence of a global industry providing potentially curative treatments targeted at conditions with high unmet medical need. This includes cancer, inherited genetic disorders and chronic degenerative diseases the increasing prevalence of which is associated with an ageing population. The global cell and gene therapy industry is growing rapidly with over 900 therapy developers sponsoring >1000 clinical trials including almost 100 phase III trials across oncology, gastroenterology, cardiovascular disease, central nervous system diseases, ocular and many other indications.

However, ATMPs are considerably different from existing treatments and require new ways of working by both industry and healthcare systems. Increasing complexity of products for a diverse range of clinical conditions, coupled with increased patient demand will bring new challenges. It is estimated 10 licensed products will come to market/annum over the next 5 years alongside an expanding number of clinical trials year on year. This development/expansion of ATMPs will bring challenges of scale-up and -out, and will require new extended supply chains and innovative treatment modalities necessitating unprecedented partnership between healthcare providers and industry – which can only be achieved by cooperative working between all parties from the outset. The integration of new innovation within the healthcare system has the potential to transform the current shepherded delivery to a turn-key industrialised delivery of these life-saving treatments.

UK Regulatory authorities, the NHS and Government are coming together to accelerate access for patients to these life changing medicines with initiatives such as the Advanced Therapy Treatment Centre Network and the Accelerated Access Collaborative which aim to facilitate the innovative a joint working detailed above.

**STRATEGIC OPTIONS FOR MANUFACTURING AND SUPPLY**

*Miguel Forte (BE)*

**ADVANCED THERAPIES LOGISTICS WITHIN A HEALTHCARE PROVIDER: CHALLENGES AND OPPORTUNITIES**

*Chris Herbert (US)*

Advanced Therapies are making rapid progress towards becoming commissioned as therapeutic options outside the research setting. Whilst much work has gone into shipping patient-specific starting materials and therapies around the world, internal logistics within complex hospital sites remain a challenge. Whilst the current situation is manageable for the relatively small numbers of therapies that are used in commissioned services or research, the challenge of how this would be managed at scale needs to be addressed so that an inability to manage Advanced Therapies within a hospital doesn’t become a blocker to wide scale adoption.

This talk will examine the challenges identified with the chain of custody and management of Advanced Therapies within Leeds Teaching Hospitals NHS Trust, one of the largest providers of healthcare in Europe which operates across a number of different sites. It will look at the challenges associated with product receipt from couriers, management and oversight from the pharmacy team, integration of logistics with the complex care needs of a patient and the moving and handling of received therapies through a large, complex organisation.
ISCT-CBA Cord Blood Workshop in partnership with WMDA and ASTCT

Co-Chairs: Elizabeth J. Shpall (US) & Joanne Kurtzberg (US)

ISCT and the Cord Blood Association (CBA) in partnership with WMDA and ASTCT are pleased to host the ISCT 2020 Cord Blood Workshop. Join the world’s leading cord blood experts to learn about the latest cord blood and cord-tissue derived cell sources and their recent clinical uses in immunotherapy, gene therapy and regenerative medicine for sickle cell, GVHD, ARDS and viral diseases. The impact of COVID-19 on cord blood banking will also be addressed and current Cord Tissue MSC trials highlighted.

**OPTIMIZING RESULTS OF CORD BLOOD TRANSPLANTS**

Éliane Gluckman (FR)

**CAR NK CELL THERAPY**

Katy Rezvani (US)

**CORD BLOOD DERIVED VIRUS-SPECIFIC T CELLS – BROADENING APPLICABILITY**

Catherine Bollard (US)

Learning Objectives:

- Understanding the strategies for manufacturing VSTs from cord blood
- Understand the advantages of CB VSTs over third party VSTs
- Obtain knowledge regarding the breadth of targetable viruses using CB as a donor source

**CORD BLOOD TISSUE DERIVED EXOSOMES FOR CLINICAL USE**

Mayela Mendt (US) and Elizabeth J. Shpall (US)

**CORD TISSUE MSCS, A NOVEL THERAPEUTIC CELL FOR IMMUNE MODULATION**

Joanne Kurtzberg (US)

Learning Objectives:

- Review the complexities of manufacturing cord tissue MSCs.
- Present data using these cells in children with Autism Spectrum Disorder.
- Discuss novel applications for these cells in treating COVID-ARDS

**THE EFFECT OF THE COVID PANDEMIC ON CORD BLOOD BANKING**

Heidi Elmoazzen (CA)

Learning Objective:

- To understand the impact of the COVID pandemic on cord blood banks around the world

**CORD BLOOD TISSUE DERIVED EXOSOMES FOR CLINICAL USE**

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**NEW APPROACHES IN TISSUE ENGINEERING AND CELL THERAPY IN GASTROINTESTINAL MOTILITY**

Khalil N. Bitar (US)
NEW ACQUISITIONS INTO LIVER REGENERATIVE MEDICINE: FROM STEM CELL NICHES TO CLINICAL APPLICATIONS

Vincenzo Cardinale (IT)

WHOLE-LIVER BIOENGINEERING: THE FUTURE OF TRANSPLANTATION MEDICINE

Pedro Baptista (ES)

Hot Topic Session – Gene Edited and Engineered Cell-Based Therapeutics for Cancer

Co-Chairs: Khalid Shah (US) and Massimo Dominici (IT)

Previously published studies have shown that different stem cells including MSC home to tumors and thus can be armed with therapeutic transgenes, a strategy that can be used to inhibit tumor growth by selectively inducing apoptosis in proliferating tumor cells. Recently, studies have demonstrated the translational potential of gene edited and engineered stem cells in mouse models of primary and metastatic tumors that mimic clinical settings. This proposed session aims at bringing together investigators who have gene edited and engineered stem cells and demonstrated novel pre-clinical efficacy of engineered stem cells alone or in combination with other immunomodulatory cells in mouse tumor models.

Learning Objectives:
The educational objectives of this session/symposium will be following:

- Multiple approaches in gene editing and engineering of stem cells
- The challenges of applying engineered stem cell in tumor models
- The clinical translation of therapeutic stem cells

ENGINEERING MSC AGAINST TUMORS

Massimo Dominici (IT)

GENE EDITED AND ENGINEERED EDITED CELL THERAPIES FOR SOLID TUMORS

Khalid Shah (US)

State of the Art: Success and Failure of Cell Therapy Clinical Trials for Acute and Chronic Lung Diseases

Co-Chairs: Patricia R M Rocco (BR) and Claudia Dos Santos (CA)

Mesenchymal stem/stromal cell (MSC) therapy holds promise for the treatment of acute and chronic lung diseases. The benefits of MSC-based therapies appeared to be induced by complex, well-orchestrated signaling pathways rather than by any one (or few) mechanisms. Safety results from phase I and II clinical trials are encouraging, but the safety and efficacy profile has yet to be proven in large-scale trials. In an ideal clinical scenario, MSCs would be promptly available and obtained through well-standardized procedures, but some barriers to the feasibility of MSC therapy still exist. In this symposium, we will discuss the current “state of the art” in success and failure of cell-therapy clinical trials for acute and chronic lung diseases. The ultimate goal is to establish an international framework for collaboration between clinical trialists, translational scientists, and the industry to serve as a platform for collaboration and advancement of clinical research in acute and chronic lung diseases.

THE GLOBAL IMPACT OF CELL THERAPY FOR ACUTE AND CHRONIC LUNG DISEASES

Daniel J. Weiss (US)

CELL THERAPIES FOR LUNG DISEASES: BUILDING ON PRECLINICAL EVIDENCE TOWARDS SUCCESSFUL CLINICAL TRANSLATION

Shirley Mei (CA)

BONE MARROW DERIVED ADULT STEM CELLS (MAPCS) FOR ARDS

Anthony Ting (US)

LESSONS LEARNED AND HOW TO IMPROVE FUTURE CLINICAL TRIAL DESIGN

Bernard Thebaud (CA)
Co-Chairs: Ivan Martin (CH) and Joan García (ES)

The field of regenerative medicine has shown tremendous growth in just a few short years, with advancements at all stages of translation for cell-based therapeutics. This session will highlight important considerations and recent steps taken to improve the safety and efficacy of regenerative medicine products as the CGT field continues to rapidly expand. Harmonization of terminology, labeling, and point of care manufacturing will be discussed followed by a look into the importance of registries and real-world data.

POC MANUFACTURING OF MSC
Fermin Sánchez-Guijo (ES)

SAFETY OF REGENERATIVE TISSUE PRODUCTS, TRACEABILITY AND OTHER SAFETY ISSUES
Ineke Slaper-Cortenbach (NL)

INTRODUCTION TO THE RM TERMINOLOGY
Marina Maréchal (BE)

Chair: Brooke Helfer (US)

Speakers will present their experience with imaging cellular therapeutics to bring awareness to how non-invasive in-vivo cell tracking technologies and methods can provide unique opportunities to optimize efficacy of cell-based therapies and aid in the assessment and management of eventual toxicities, and ultimately, benefit their clinical translation.

Learning objectives:
• To learn about the different imaging modalities for monitoring cellular therapies
• To learn about developing and available, clinically applicable cell tracking technologies
• To learn how imaging can aid in the translation of cellular therapeutics
MONITORING INTRACEREBELLAR DELIVERY OF NATURAL KILLER CELLS
Vidya Gopalakrishnan (US)

Gene Therapy for Globin Disorders
Chair: Sandeep Soni (US)
Gene therapy of globin disorders is one of next big things to hit the field, due to the size of the candidate populations, the unmet demand that it represents, rapid technological evolutions, and the foreseeable enormous challenges in terms of market access and financial sustainability. This session will address recent advancements in gene therapy for the treatment of hemoglobinopathies and next steps to bring this innovative therapeutic approach to affected patients.

GENE THERAPY FOR B-HEMOGLOBINOPATHIES
Marina Cavazzana (FR)
Beta-thalassemia and sickle cell disease are the most prevalent monogenic diseases and are caused by quantitative or qualitative defects in the production of adult hemoglobin. Gene therapy is a potential treatment option for patients lacking an allogenic compatible hematopoietic stem cell (HSC) donor. Over the last fifteen years, gene therapy through the use of genetically modified autologous hematopoietic cells has shown in several clinical trials its powerful outcome to successfully treat globin disorders. Lastly, genome-editing and homologous recombination technologies have undergone spectacular developments over the last couple of decades. In view of the impressive progress reported for the gene-addition strategy, gene-editing approaches to patients affected by globin disorders would move gene therapy one step forward.

A SINGLE DOSE OF CD117 ANTIBODY DRUG CONJUGATE ENABLE HEMATOPOIETIC STEM CELL BASED GENE THERAPY IN NONHUMAN PRIMATES
Rahul Palchaudhuri (US)
This presentation will summarize recent pre-clinical research to show proof of concept that a single dose of targeted antibody drug conjugate is sufficient to enable HSC-based gene transfer in relevant nonhuman primate model without the need for chemo or radiotherapy.

GENE-EDITING FOR HEMOGLOBINOPATHIES
Sandeep Soni (US)
The objectives of this talk are to describe the various gene-editing platforms currently being investigated for hemoglobinopathies. The presentation will also highlight the differences between gene-insertion and gene-editing platforms and provide an overview of the current trials.

Oral Abstract Showcase
Chair: Daniel J. Weiss (US)

ORAL ABSTRACT PRESENTERS:

GENE EDITING USING CRISPR ENABLES FOXP3 GENE REPAIR IN HSPCS AND IPEX PATIENT T CELLS (Abstract O22)
Esmond Lee (US)

SAFE AND EFFECTIVE TREATMENT OF ACUTE LIVER FAILURE BY ALLOGENEIC TRANSPLANTATION OF STEM CELL-DERIVED ENCAPSULATED LIVER TISSUE WITHOUT IMMUNOSUPPRESSION (Abstract O24)
Massimiliano Paganelli (IT)

MAGNETIC 3D BIOPRINTING FOR PERSONALIZED MEDICINE (Abstract O23)
Glauco Souza (US)

PREDICTIVE MODELING DEMONSTRATING A TWO-FACTOR SIGNATURE AS EARLY AS A WEEK PRIOR TO HARVEST FOR CHIMERIC ANTIGEN RECEPTOR (CAR) T-CELL MANUFACTURING GROWTH (Abstract O26)
Jun Xu (US)

PROBING MSC AND TUMOR CELL SECRETOME LOCALLY VIA DYNAMIC SAMPLING PLATFORM (DSP) (Abstract O10)
Mason Chilmonczyk (US)

ENGINEERING OF NK ACTivating RECEPTOR LIGANDS ENHANCES IMMUNE COMPATIBILITY OF MHC-I/- IPSC-DERIVED β CELLS FOR CELL THERAPY OF TYPE 1 DIABETES (Abstract O11)
Raniero Chimienti (IT)
SESSION SUMMARIES

ENHANCING THE THERAPEUTIC POTENTIAL OF MESENCHYMAL STEM CELL-BASED THERAPY VIA CRISPR/CAS9-BASED GENOME EDITING (Abstract O12)
Jae Young Lee (KR)

ACOUSTIC AFFINITY CELL SELECTION: A NON-PARAMAGNETIC SCALABLE TECHNOLOGY FOR T CELL SELECTION FROM UNPROCESSED APHERESIS PRODUCTS (Abstract O14)
Rui Tostoes (US)

MESENCHYMAL STEM CELL THERAPY IMPROVES PULMONARY FUNCTION AND EXERCISE TOLERANCE IN PATIENTS WITH CHRONIC OBSTRUCTIVE PULMONARY DISEASE (COPD) AND HIGH BASELINE INFLAMMATION (Abstract O36)
Jack Hayes (US)

FAST AND SERIOUS: COMPARATIVE STUDY OF THE IMPACT OF ACCELERATED PROCEDURE DESIGNATIONS ON MARKET ACCESS FOR ADVANCE THERAPY MEDICINAL PRODUCTS (Abstract O13)
Baptiste Pileyre (FR)

BONOFILL-II, FROM BENCH TO BEDSIDE: A NOVEL AUTOLOGOUS CELL-BASED, TISSUE-ENGINEERED PRODUCT IN LINE TO REPLACE BONE AUTOGRRAFTS FOR LARGE SEGMENTAL BONE DEFECT APPLICATIONS (Abstract O23)
Dror Ben-David (IL)

The provision of unproven cell interventions sold directly to consumers remains a global challenge, posing risks to patients and complicated the development of safe and effective proven cell therapies. Indeed, despite, or perhaps because of, increased scrutiny from regulatory bodies, the unproven cell therapy industry has evolved rapidly, marketing new interventions, including unproven exosome-based products, and taking advantage of various regulatory pathways, such as expanded access and right-to-try. This panel brings together multiple perspectives on the development of unproven cell therapies to bring audience members up to date on emerging trends in this industry and on the activities and plans of the ISCT Presidential Task Force on the Use of Unproven and/or Unethical Cell and Gene Therapy.

ETHICAL AND POLICY CONSIDERATIONS ASSOCIATED WITH SPECULATIVE CELL BANKING SERVICES
Aaron Levine (US)

EXTRACELLULAR VESICLE-BASED UNPROVEN THERAPIES
Eva Rohde (AT)

SITUATING STEM CELLS WITHIN THE REGULATORY ENVIRONMENT FOR PATIENT-DRIVEN ACCESS
Patricia J. Zettler (US)

ISCT PRESIDENTIAL TASK FORCE ON THE USE OF UNPROVEN AND UNETHICAL CELL & GENE THERAPIES ANNUAL GENERAL MEETING
Massimo Dominici (IT) and Laertis Ikonomou (US)

Optimizing a product through process development is a natural step in translating the therapeutic to the clinic. While this is a critical milestone, many of the processes have not been optimized for larger-scale industrialization. This session will evaluate how industry innovators are implementing new processing platforms and strategies to position future products for sustainable industrialization.

ISCT Presidential Task Force Session - Emerging Issues in Unproven Cell Therapies

Co-Chairs: Laertis Ikonomou (US) Aaron Levine (US)
CHALENGES IN TRANSLATING ACADEMIC AND START-UP COMPANIES TO FIRST IN MAN AND EARLY PHASE CLINICAL TRIALS

Stuart Curbishley (UK)

We will discuss the challenges in translating academic and start-up companies to first in man and early phase clinical trials. In particular we will explore the experience of the Birmingham group in embedding a European academic spin-out into their GMP manufacturing facility and equipping the company to develop its own manufacturing expertise. Furthermore, we will discuss the outcomes of the Innovate UK funded Advanced Therapy Treatment Centres and how this initiative is improving the logistics of ATMP manufacture and distribution.

ALLOGENEIC T-CELL THERAPIES: SHIFTING TOWARD COMMERCIAL MANUFACTURING

Jean-François Chaubard (BE)

SURVIVAL GUIDE TO CDMO

David Smith (US)

**Strategies for Commercialization Track Session - Can We Industrialize CAR T Therapies? The Practical Challenges in Achieving Wide-Spread Patient Access**

Chair: William Milligan (CA/TW)

Today the first two autologous CAR-T therapies are commercialized in multiple developed countries, through dozens of health care facilities, and many hundreds of patients are benefiting from treatment! Now “industrializing” CAR-T therapy to gain world-wide, broad patient access to this new standard of care, means not only extensive scale-up of manufacturing capacity but also transforming hundreds of health care centers into CAR-T competent treatment sites using either autologous or “off-the-shelf” allogeneic products in treating patients. What are some key considerations for how academia, industry and healthcare centers can meet this challenge? Today we’ll explore autologous, allogeneic, and healthcare center perspectives from four experts active in pursuing this CAR-T industrialization vision.

HOW TO COMMERCIALIZE AUTOLOGOUS CAR-TS

Gunther Busam (CH)

Learning objectives:
- Considerations for successful commercialization of autologous CAR-Ts
- Manufacturing Network strategy and Apheresis network
- Preparation of Supply Network during clinical development
- Challenges and Opportunities for autologous CAR-Ts

INDUSTRIALIZING CAR-TS: WHAT CAN WE LEARN FROM 50+ YEARS OF STEM CELL TRANSPLANTATION?

Elizabeth Hexner (US)

Learning objectives:
- Challenges and advantages of both allogeneic and autologous products
- Recognize existing structures in centers with decades of cell therapy expertise
- Minimizing complexity when launching a new product

DEVELOPMENT CHALLENGES WHEN YOUR RAW MATERIAL IS HUMAN AND THE PRODUCT IS A POPULATION

Suma Rao (US)

Learning objectives:
- Introduction to allogeneic process and comparison vs autologous
- Challenges in delivering robust control strategy due to:
  - Complex and new raw materials
  - Emerging technologies in manufacturing and detection
  - Single sourced materials and equipment
- Challenges associated with product definition when the product is not an entity but a population and the use of Quality Target Product Profile as a tool to address challenge
DEVELOPING ALLOGENEIC CANCER IMMUNOTHERAPY WITH IPSC TECHNOLOGY

Wen Bo Wang (US)

Learning objectives:
- Pioneering a revolutionary approach using renewable master induced pluripotent stem cell (iPSC) lines generated from our proprietary iPSC platform to derive cell therapy product candidates that can be delivered off-the-shelf for the treatment of a large number of patients.
- Our cell therapy product candidate pipeline is comprised of immuno-oncology programs, including off-the-shelf NK- and T-cell product candidates derived from master iPSC lines.
- Challenges in cell culture scale up for allogeneic cell therapies with iPSC technology.

Strategies for Commercialization Track Session – Clinical Experience in Regenerative Medicine and Tissue Engineering, The Next Wave of Advanced Therapies

Chair: Julie Allickson (US)

CLINICAL TRANSLATION OF TISSUE ENGINEERING IN AN ACADEMIC FACILITY

Julie Allickson (US)

PERSONALIZED TISSUE-ENGINEERED ORGANS THAT WILL REVOLUTIONIZE FUTURE MEDICINE

Petter Björquist (SE)

The severe and incurable stages of Chronic Venous Insufficiency (CVI) currently affect at least 1.5M patients in the EU and North America alone, with an approximately 300,000 new patients diagnosed every year. The disease significantly impacts quality of life and puts numerous patients out of work or into disability programs. CVI is a major burden to patients, employers, and health care systems. VERIGRAFT estimates that its first product, the personalised tissue-engineered vein (P-TEV), has the potential to cure patients with severe CVI and enable them to return to a normal life.

VERIGRAFT is just about to initiate a unique clinical trial program where P-TEV will be used to treat CVI. We have received approval for the first trial by the European authorities and are preparing for a pre-IND meeting in the US. The trial in Europe will be first of its kind worldwide, and one of the first tissue-engineered products clinically tested in Europe. The presentation will describe the road from research and an unmet medical need to a market authorization for this novel ATMP. Personalization and tissue-engineering of some other organs will also be mentioned.

Key items of the presentation:
- Generation of personalized tissue-engineered transplants
- Use of regenerative medicine to address so far incurable diseases
- Organ transplantation without the severe risks of immunosuppression
- Industrialization of a tissue-engineered ATMP, from bench to bedside

WILL ENGINEERED TISSUES TRANSFORM MEDICINE?

Laura Niklason (US)

Quality and Operations Track Session – Qualification, Regulation of Gene Editing Tools in Cell & Gene Therapy

Chair: Shirley Bartido (US)

Recent advances in genome editing technologies have substantially improved our ability to make changes in the genomes of eukaryotic cells. Viral vectors and programmable nucleases are already revolutionizing our ability to interrogate the function of the genome. This session provides an overview of current progress in qualifying and regulating targeted genome editing technologies as they are being used clinically to correct or introduce genetic mutations to treat diseases that are refractory to traditional therapies. Preclinical assessment and GMP manufacturing of these technologies as well as the regulatory requirements for FIH clinical trials utilizing these technologies will be discussed.
IMPAKT OF THE USE OF HEALTHY VS DISEASED DONORS ON THE DEVELOPMENT OF T CELL THERAPIES

Corey Smith (AU)

The presentation will discuss the approaches we have used at QIMR Berghofer to develop T cell immunotherapies to target viral infection in immunocompromised patients, cancer patients and patients with autoimmune disease. It will discuss some of the lessons we have learned from the transition from healthy donors for process optimization and validation to the use of patient material in manufacturing that may be shipped from around Australia or overseas.

EXAMPLES OF PROCESS VALIDATION OF THE AUTOLOGOUS VS ALLOGENEIC: SCHWANN CELLS VS MESENCHYMAL STEM CELLS DERIVED EXOSOMES

Aisha Khan (US)

Process validation means a successful demonstration of manufacturing and quality consistency, and it is the action of providing that any process, procedure, method, or activity actually and consistently fulfill specific requirements. This session will outline the process validation steps by using examples of autologous and allogenic products.

Learning objectives:
• Understanding and using FDA’s Process Validation Guideline
• Is process validation for autologous is different than allogeneic?
• How to validate process for Investigational New Drug (IND) applications?
• Establishing and maintaining control of complex processes, as well as achieving regulatory approval of new products.
• Understanding the importance of process validation as a means of minimizing the concerns related to cell manufacturing.
• Monitoring, process changes and when to revalidate.
Quality and Operations Track
Session - Characterization of Active Substance and Comparability

Chair: Rosemarie Bell (AU)

Making changes to the manufacturing process/product is an inevitable part of process development with the goal of making a better product. There is a risk if manufacturing changes are made late in the clinical trials that they could potentially change the product's critical characteristics.

Foreign regulatory authorities as well as FDA have broadly adopted the requirements defined in ICH Q5E: Comparability of Biotechnological/Biological Products Subject to Changes in Their Manufacturing Process for establishing product comparability. The comparability study is defined as a prospective study protocol designed to demonstrate that two products are comparable before and after the change. In some cases, the Agency may ask the IND sponsor or applicant to submit the comparability study for assessment and review prior to the data collection and analysis.

In ICH Q5E comparability is defined as a conclusion that products are highly similar before and after manufacturing process changes with no predicted adverse impact on the quality, safety, or efficacy of the drug product. This conclusion is most often based on an analysis of product quality attributes. In some cases where subtle analytical changes are seen, nonclinical or even clinical/immunogenicity data might be indicated. When this document was written in the 1990’s, biological (biotech) products were within scope, unfortunately cell and gene therapy products were not because this product sector was in its infancy.

This session will focus on providing stakeholders with some guidance about best practices to follow to demonstrate comparability and major consideration and essential element of well-designed and executed comparability studies.

APPROACHES TO COMPARABILITY STUDIES OF CAR-T PRODUCTS
Mehrshid Alai-Safar (US)

Comparability studies of genetically modified autologous cell therapy products could be challenging in many aspects. Different approaches and limitations to each will be discussed.

ESSENTIAL ELEMENTS OF BEST PRACTICES TO FOLLOW FOR DEMONSTRATING PRODUCT COMPARABILITY
Mo Heidaran (US)

THE ROLE OF BIG-DATA ANALYTICS IN DEVELOPING PREDICTIVE CQAS AND CPPS, AND ENABLE REAL-TIME PROCESS MONITORING, FOR CELL THERAPY MANUFACTURING
Krishnendu Roy (US)

This talk will outline how multi-omics based big-data analytics and multivariate predictive modeling can be used to better understand cell manufacturing processes and develop putative CQAs, early predictive markers, and identify critical process parameters to enable product and process comparability and understand mechanism of actions.
INVITED CHAIRS & SPEAKERS

Mehrshid Alai-Safer, PhD, Kite Pharmaceuticals, United States
Julie Allickson, PhD, MS, MT(ASCP), Wake Forest Institute for Regenerative Medicine, United States
Pedro M. Baptista, PharmD, PhD, IIS Aragon, Spain
Jacqueline Barry, PhD, Cell and Gene Therapy Catapult, United Kingdom
Shirley Bartido, PhD, MBA, Cellectis, United States
Patrick Bedford, MBHL, RAC, weCANreg Consulting Group Inc., Canada
Rosemarie Bell, B.App.Sc Micro/Biochem MASM, Q-Gen Cell Therapeutics QIMR Berghofer Medical Research Institute, Australia
Khalil Bitar, PhD, AGAF, MBAe, CELLF BIO LLC, United States
Petter Björquist, PhD, VERICRAFT AB, Sweden
Catherine Bollard, MBCHB, MD, Children’s National Hospital, United States
Halvard Bönig, MD, Goethe University Medical School, Germany
Christopher Bravery, PhD, Consulting on Advanced Biologicals, United Kingdom
Scott Burger, MD, Advanced Cell & Gene Therapy, United States
Gunther Busam, RPh, PhD, Celgene International Sàrl, Switzerland
Lizette Caballero, MLS(ASCP), HemaCare Corporation, United States
Maria Cancio, MD, Memorial Sloan Kettering Cancer Center, United States
Vincenzo Cardinale, MD, PhD, Sapienza University of Rome, Italy
Marina Cavazzana, MD, PhD, Assistance Publique - Hôpitaux de Paris, France
Christian Chabannon, MD, PhD, Institut Paoli Calmettes and Aix-Marseille Université, France
Jean-François Chaubard, MSc, MaSTherCell, Belgium
Dominic Clarke, PhD, HemaCare Corporation, United States
Enrico Clini, MD, University of Modena and Reggio Emilia, Italy
Stuart Curbishley, PhD, University of Birmingham Medical School, United Kingdom
David DiGiusto, PhD, Semma Therapeutics, United States
Massimo Dominici, MD, University Hospital of Modena & Reggio Emilia - Modena Policlinic, Italy
Claudia Dos Santos, MD, University of Toronto and St. Michael’s Hospital, Canada
Melanie Eacho, PhD, CBER/FDA, Cell Therapy Branch, Division of Cell and Gene Therapies, United States
Heidi Elmoazzen, PhD, Canadian Blood Services, Canada
Dominique Farge, MD, PhD, St-Louis Hospital, AP-HP
Paul S. Frenette, MD, Albert Einstein College of Medicine, United States
Jacques Galipeau, MD, FRCP(C), University of Wisconsin-Madison, United States
Joan Garcia, MD, PhD, Banca De Sang I Teixits, Spain
Bernd Giebel, PhD, University of Duisburg-Essen, Germany
Mario Gimona, PhD, Paracelsus Medical University Salzburg, Austria
Élaine Gluckman, MD, FRCP, Eurocord, France
Massimiliano Gnocchi, MD, PhD, University of Pavia & IRCCS Policlinico San Matteo, Italy
Vidya Gopalakrishnan, PhD, MD Anderson Cancer Center, United States
Ronald J. Hause, PhD, june Therapeutics, a Bristol-Myers Squibb company, United States
Mo Heidarani, PhD, Parexel International, United States
Brooke Helfer, PhD, Celense Inc., United States
An Hendrix, PhD, Ghent University, Belgium
Christopher Herbert, PhD, Leeds Teaching Hospitals NHS Trust, United Kingdom
Elizabeth Hexner, MD, MSTR, University of Pennsylvania, United States
Tobias Hohl, MD, PhD, Memorial Sloan Kettering Cancer Center, United States
Emily Hopewell, PhD, Indiana University School of Medicine, United States
Michael Hudecek, MD, Universitätsklinikum Würzburg, Germany
Laertis Ikonomou, MD, PhD, University at Buffalo, The State University of New York, United States
Rudolf Jaenisch, MD, Whitehead Institute and Massachusetts Institute of Technology, United States
David Jones, BSc, MSc, EurBioI, CBioI, FRSB, MTOPRA, ERT, FBTS, MHRA, United Kingdom
Christian Jorgensen, MD, PhD, University Montpellier, INSERM, France
Diane Kadidlo, BS, CLS (ASCP), Molecular and Cellular Therapeutics University of Minnesota, United States
Aisha Khan, MSc, MBA, University of Miami, United States
Maroun Khoury, PhD, Cells for Cells - REGENERO, Chile
Donald Kohn, MD, University of California, Los Angeles, United States
Angela Krackhardt, MD, Technical University of Munich, Germany
Mauro Krampera, MD, PhD, Section of Hematology and Bone Marrow Transplant Unit, Department of Medicine, University of Verona, Italy
Joanne Kurtzberg, MD, Duke University Medical Center, United States
John Laffey, MD, MA, FCAI, FJICMI, NUI Galway, Ireland
Aaron Levine, PhD, Mphil, Georgia Tech, United States
INVITED CHAIRS & SPEAKERS

Sai-Kiang Lim, PhD, A*STAR Institute of Medical Biology, Singapore
Janet Macpherson, PhD, Cytiva, Australia
Marina Maréchal, DDS, PhD, KU Leuven, Belgium
Ivan Martin, MD, University Hospital Basel, Switzerland
Marcela Maus, MD, PhD, Massachusetts General Hospital, United States
Shirley Mei, PhD, MSc, Ottawa Hospital Research Institute, Canada
Philippe Menasché, MD, Hôpital Européen Georges-Pompidou, Paris, France
Mayela Mendt, PhD, MD Anderson Cancer Center, United States
William Milligan, Steminent Biotherapeutics Inc., Canada
Julie Murrell, PhD, MilliporeSigma, United States
George Muschler, MD, Cleveland Clinic, United States
Vivek K. Narayan, MD, MSC, University of Pennsylvania, Abramson Cancer Center, United States
Karen Nichols, Esq, United States
Laura Niklason, MD, PhD, Yale University and Humacyte Inc., United States
Charles A. O’Neill, PhD, DABT, BioMarin Pharmaceuticals Inc., United States
Giuseppe Orlando, MD, PhD, Wake Forest University, United States
Emanuele Ostuni, PhD, Novartis Oncology, Switzerland
Rahul Palchaudhuri, PhD, Magenta Therapeutics, United States
Arnaud Paris, BSc, bioMérieux, France
Marc Peschanski, MD, PhD, I-Stem, France
Nicolas Piuuzzi, MD, Cleveland Clinic, United States
Suma Rao, PhD, Allogene Therapeutics, United States
John Rasko, AO, BSc(Med), MBBS(Hons), PhD, MAICD, FFSc(RCPA), FRCPA, FRACP, FAHMS, University of Sydney, Australia
Stephan Reynier, MSc, Celletics, France
Katy Rezvani, MD, PhD, MD Anderson Cancer Center, United States
Paula Río, PhD, Centro de Investigaciones Energéticas, medioambientales y Tecnológicas (CIEMAT), Spain
Patricia R. M. Rocco, MD, PhD, Federal University of Rio de Janeiro, Brazil
Scott Rodeo, MD, Hospital for Special Surgery, United States
Eva Rohde, MD, Paracelsus Medical University Salzburg, Austria
Krishnendu Roy, PhD, NSF Engineering Research Center (ERC) for Cell Manufacturing Technologies (CMaT), United States
Antonio Ruiz-Garcia, MP Pharm, Hospital Universitario Virgen de las Nieves, Spain
Fermin Sánchez-Guijo, MD, PhD, IBSAL-University Hospital, University of Salamanca, Spain
Alberto Santagostino, MBA, Lonza AG, Switzerland
Khalid Shah, MS, PhD, Harvard Medical School, United States
Yufang Shi, PhD, Shanghai Institutes for Biological Sciences, United States
Elizabeth J. Shpall, MD, The University of Texas MD Anderson Cancer Center, United States
Donald Singer, MS, Ecolab, United States
Ineke Slaper-Cortenbach, PhD, University Medical Center Utrecht, Netherlands
Corey Smith, PhD, QIMR Berghofer Medical Research Institute, Australia
David Smith, MBA, Akron, United States
Sandeep Soni, MD, Stanford University, United States
Jane Sosabowski, PhD, Queen Mary University of London, United Kingdom
Karim Tarte, PharmD, PhD, UMR INSERM U1236, CHU Rennes, EFS Bretagne, France
Bernard Thébaud, MD, Ottawa Hospital Research Institute, Canada
Clotilde Théry, PhD, INSERM/Institut Curie, France
Anthony Ting, PhD, Athersys Inc., United States
Basak Uygun, PhD, Massachusetts General Hospital and Harvard Medical School, United States
Santhosh Vardhana, Memorial Sloan Kettering Cancer Center, United States
Sowmya Viswanathan, PhD, University Health Network, Canada
Dominic Wall, PhD, FFSc(RCPA), Cell Therapies Pty Ltd, Peter MacCallum Cancer Center, Australia
Wen Bo Wang, PhD, Fate Therapeutics, United States
Bettina Weigelin, PhD, Werner Siemens Imaging Center, University of Tubingen, Germany
Daniel J. Weiss, MD, PhD, University of Vermont, United States
Colin White, PhD, Vertex Pharmaceuticals, United States
Adrian Woolson, BM.,B.Ch, PhD, Sangamo Therapeutics, Inc., United States
Peter Zandstra, PhD, FRSc, PEng, University of British Columbia, CCRM, ExcelliVera, Canada
Patricia Zettler, J.D., The Ohio State University Moritz College of Law, United States
ABSTRACT AWARD WINNERS

Top Scoring Abstract Award

Anusha Shankar, MSc, MD, Erasmus Medical Center, Rotterdam, Netherlands

- **Abstract #O3**: Human Kidney Organoids Produce Functional Renin
- **Presentation**: May 28, Plenary Satellite – iPSC for Cardiovascular Regenerative Medicine & Disease Modelling

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Early Stage Professional Awards

Jun Xu, PhD, Perelman School of Medicine at the University of Pennsylvania; Center for Cellular Immunotherapies, United States

- **Abstract #O26**: Predictive Modeling Demonstrating a Two-Factor Signature as Early as a Week Prior to Harvest For Chimeric Antigen Receptor (CAR) T-Cell Manufacturing Growth
- **Presentation**: Pre-Recorded, ISCT 2020 Oral Abstract Showcase

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NEW! ESP Rookie of the Year Award

Jae Young Lee, BEng, PhD, ToolGen, Korea (the Republic of)

- **Abstract #O12**: Enhancing the Therapeutic Potential of Mesenchymal Stem Cell-Based Therapy via CRISPR/Cas9-Based Genome Editing
- **Presentation**: Pre-Recorded, ISCT 2020 Oral Abstract Showcase

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AWARD WINNERS

Technologist Awards

**Rui Tostoes, PhD, MilliporeSigma, United States**
- Abstract #O14: Acoustic Affinity Cell Selection: a Non-Paramagnetic Scalable Technology for T Cell Selection from Unprocessed Apheresis Products
- Presentation: Pre-Recorded, ISCT 2020 Oral Abstract Showcase

**Zhuoer Lin, PhD, Janssen, JnJ, United States**
- Abstract #P28: Development of a Closed Wash/Formulation Process for CAR-T Drug Product
- Presentation: E-Poster, Dedicated Poster Hall Presenting Hours (North America/South & Central America)

**Hyunjung Park, PhD, CHA Stem Cell, Korea (the Republic of)**
- Abstract #O29: The Genetically-Engineered Stem Cell Therapy of Huntington Disease: SPT4 Knockout HD Patient IPSC-NPCS Transplantation Rescue Abnormal Neuronal Dysfunction in the YAC128 Model
- Presentation: May 28, Plenary Satellite – Gene Therapy Clinical Trials

Emerging Economy Abstract Award

**Hossein Baharvand, PhD, Royan Institute, Iran (the Islamic Republic of)**
- Abstract #P2: Human ES Cell-Derived Dopaminergic Transplants Function in a Primate Model of Parkinson’s Disease
- Presentation: E-Poster, Dedicated Poster Hall Presenting Hours (Europe/Asia Pacific)

NEW! ISCT ANZ Early Stage Professional Travel Award

**Dandan Zhu, PhD, MD, Hudson Institute of Medical Research, Australia**
- Abstract #O35: Prematurity Negatively Impacts Therapeutic Effect of Human Amnion Epithelial Cells in Experimental Bronchopulmonary Dysplasia
- Presentation: Pre-Recorded, Plenary Satellite – MSC-EV Technical Considerations
NEW! Top Scoring Immunomodulation Abstract Award

Xiaoran Zhang, PhD, Sun Yat-Sun University, China

- Abstract #O7: Mesenchymal Stromal Cells Alleviates Experimental Acute Respiratory Distress Syndrome through the Cholinergic Anti-Inflammatory Pathway
- Presentation: May 29, Plenary Satellite - From Mechanism of Action to Establishing Collaborative Networks: The Opportunities, Challenges and Tangible Progress in the Development of Cell-Based Therapies for Musculoskeletal Disease

Award Sponsored by:

NEW! Top Scoring Musculoskeletal Repair & Regeneration Abstract Award

Karoliina Pelttari, PhD, University Hospital Basel and University of Basel, Switzerland

- Abstract #O9: Engineered nasal cartilage for the repair of osteoarthritic knee cartilage defects
- Presentation: May 29, Plenary Satellite - From Mechanism of Action to Establishing Collaborative Networks: The Opportunities, Challenges and Tangible Progress in the Development of Cell-Based Therapies for Musculoskeletal Disease

Award Sponsored by:

NEW! Top Scoring Immunomodulation Abstract Award

Philip Lee, PhD, Senti Biosciences, United States

- Abstract #O6: Manufacturing Development of SENTI-101, a Gene Circuit Modified Allogeneic Bone Marrow Derived Mesenchymal Stromal Cell (BM-MSC) Therapy for the Treatment of Solid Tumors
- Presentation: May 29, Plenary Satellite - From Mechanism of Action to Establishing Collaborative Networks: The Opportunities, Challenges and Tangible Progress in the Development of Cell-Based Therapies for Musculoskeletal Disease

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Hematopoietic stem cell (HSC) gene therapy is a promising treatment option for various hematological diseases and disorders. Most currently available approaches target CD34+ cell–enriched fractions, a heterogeneous mix of mostly committed progenitor cells and only very few true HSCs with long-term multilineage engraftment potential. Consequently, gene therapy approaches are limited in their HSC targeting efficiency and very expensive due to the large quantities of modifying reagents needed. In addition, exposure of non-target cells to lentiviral vectors or nucleases can increase the risk of unwanted side-effects in these cell populations.

We aimed to develop a clinical protocol to reliably purify and efficiently gene-modify human HSC-enriched CD90+ cell fractions, which ultimately would allow us to reduce costs without compromising in vivo engraftment.

Large-scale enrichment of CD34+ cells from GCSF-mobilized leukapheresis products was initially performed on Miltenyi Biotec’s CliniMACS Prodigy®. Yield, purity, quality, and feasibility of CD90+ cell sorting was then tested on the jet-in-air sorter FX500 from Sony and the cartridge-based closed-system sorter MACSQuant® Tyto® from Miltenyi Biotec. Transduction was performed using a clinical-grade, GFP-encoding lentivirus. Engraftment was tested using the NSG mouse xenograft model.

Purity and yield after flow cytometric sorting of CD90+ cells were similar with either the FX500 or MACSQuant® Tyto®. Both approaches reliably reduced the overall target cell count by 10- to 15-fold without impacting the cells’ viability and in vitro colony-forming cell potential. Transduction efficiency of sorted CD90+ cells was significantly improved compared to bulk CD34+ and especially the CD34+CD90+ subset. All cell fractions demonstrated robust mouse xenograft potential. Significantly higher levels of GFP expression in peripheral blood, bone marrow, spleen, and thymus were observed after transplantation of gene-modified CD90+ compared to bulk CD34+ cells in NSG mice.

NKG2D is a C-type lectin-like transmembrane activating receptor present on the surface of natural killer (NK) cells, NKT cells, CD8+TCRγδ T cells, and certain subsets of CD4+ T cells. Biogenesis of NKG2D ligands (NKG2DL) is stimulated in cells under stress conditions such as viral infection, cellular senescence, and tumorigenesis. NKG2DL are expressed on various tumor types including different pediatric solid and hematological malignancies, thus providing suitable targets for cancer therapy. NKG2D activation on NK cells results in cytokine secretion and exocytosis of cytotoxic granules. NKG2D is particularly relevant for cancer immunosurveillance: Interaction between
NKG2DL and NKG2D receptor is essential for NK cell–mediated elimination of osteosarcoma tumor-initiating cells. However, tumor cells can develop various immune escape strategies. Nevertheless, the use of NKG2D-CAR on memory CD45RA– T cells may overcome these limitations. We have shown that NKG2D-CAR–expressing CD45RA– T cells were cytotoxic against three osteosarcoma cell lines and 8/10 leukemia cell lines with specific lysis of over 50%. Myeloid and T-ALL cell lines were more susceptible (specific lysis ranging from 50–78%) than B-ALL cell lines (19–52%). NKG2D-CAR+ memory CD45RA– T cells also had considerable antitumor activity in a mouse model of human osteosarcoma, whereas non-transduced T cells were ineffective. We have developed a protocol to expand clinical-grade NKG2D-CAR–expressing memory CD45RA– T cells in a fully automated closed system, CliniMACS Prodigy®. This expansion protocol allowed us to obtain up to 2076±697 million cells with 77.8±20% NKG2D-CAR expression and 76±10% viability. Harvested CAR T cells showed specific lysis of Jurkat cells (90±14%) and 531MII osteosarcoma cell line (31±16%). Vector copy number was ≤5 in all validations. CGH and karyotype showed no genetic alterations. Free viral particles were undetectable in the supernatants. No overexpression of MYC/TERT was found except for one validation. Endotoxins were ≤0.25 EU/mL. Automated manufacturing of clinical-grade NKG2D-CAR–expressing memory CD45RA– T cells using the CliniMACS Prodigy® is feasible and reproducible. We plan to explore different clinical trials on pediatric diseases.

MANUFACTURING OF INDUCED PLURIPOTENT CELLS AND IMMUNE CELLS FOR ADOPTIVE IMMUNOTHERAPY OF CANCER

Annelise Bennaceur Griscelli, MD, University Paris Saclay – Inserm UMR 935, France

The breakthrough discovery of induced pluripotent stem cells (iPSCs) is currently profoundly modifying the landscape of cell therapy and allows us to open novel perspectives for the generation of advanced therapy medicinal products (ATMP) potentially applicable in all fields of medicine. In terms of a large-scale therapeutic landscape, implementation of iPSC-derived allogeneic therapies will require suitable immune-HLA-matched iPSC lines from healthy universal donors and/or the derivation of hypoimmunogenic iPSC lines. To anticipate future demands in effective allogeneic therapies, the availability of accessible cost-effective and safe iPSC lines is a major requirement, to provide off-the-shelf unlimited numbers of therapeutic products from a single iPSC master cell bank. We have previously developed several research-grade master banks of human iPSCs using an in vitro expansion workflow. However, several hurdles remain for industrial, cGMP-grade, large-scale production and banking of these cells.

We report a procedure using the integrated GMP-compliant cell processing platform CliniMACS Prodigy®, providing automated cell feeding and harvesting in a closed system. The iPSC clone used on the CliniMACS Prodigy Platform was previously derived from a healthy donor using the CytoTune™- iPS 2.0 Sendai Reprogramming Kit, manufactured according to GMP principles, on StemMACS™ iPS-Brew XF, human medium and human recombinant laminin matrix. Over 14 in vitro cell passages, this cell line was replated and expanded on the CliniMACS Prodigy Platform within 2 weeks. At the end of the process, 1.4 billion iPSCs were collected with a high genetic stability as evaluated by karyotyping and CNV/NGS analysis before and after scalable expansion. Expanded iPSCs maintained their pluripotency markers and an efficient differentiation towards endodermal, mesodermal, and ectodermal trilinesage layers. Overall, this process provides a safe and standardized scalable manufacturing platform for GMP-iPSC master cell banks. The feasibility of this procedure opens novel perspectives in large-scale production of mesenchymal stem cells and immunocompetent cells for our therapeutic program.
On completion of this ISCT symposium activity, participants will be able to:

- Understand the biological/preclinical differences between current chimeric antigen receptor (CAR)-T cell therapy options for relapsed/refractory diffuse large B-cell lymphoma, as well as innovations in CAR-T cell therapy under investigation
- Recognize the role of key biomarkers and baseline characteristics in patient selection for CAR-T cell therapy treatment and patient monitoring post-CAR-T cell infusion
- Discuss the impact (or lack thereof) of CAR-T cell product attributes on safety and efficacy outcomes

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**THERAPEUTIC OPTIONS FOR CAR-T CELL THERAPY IN DLBCL**

Uwe Platzbecker, MD, University Hospital Leipzig, Leipzig, Germany

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**INNOVATIONS IN CAR-T CELL THERAPY**

Nina Worel, MD, University of Vienna, Austria

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**PREDICTORS OF CAR-T CELL THERAPY OUTCOMES IN DLBCL: BASELINE CHARACTERISTICS, BIOMARKERS, AND POSTINFUSION FACTORS**

Richard T. Maziarz, MD, Oregon Health and Science University Knight Cancer Institute, United States

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**IMPACT OF PRODUCT ATTRIBUTES ON CAR-T CELL THERAPY OUTCOMES**

Marcela V. Maus, MD, Massachusetts General Hospital Cancer Center; Harvard Medical School, United States
THE FIRST ISOLATED MANUAL FILLER

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Decontamination cycle <30min
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www.aseptictech.com
Advancing QC Testing for Cell and Gene Therapy Products
LOCATION: GLOBAL SHOWCASE THEATRE

Ali Mohamed, PhD, VP of CMC, Immatics US Inc, United States
Félix A. Montero Julian, PhD, Healthcare Scientific Affairs Global Director, bioMérieux, France
Dominic Wall, PhD FFSc (RCPA), Executive Director of Business Ventures, Chief Scientific Officer, Cell Therapies Pty Ltd, Australia

The successful development and commercialization of Cell and Gene Therapy products opened a hope for patients with urgent medical needs and opened a door for a new era of modern medicine.

However, the manufacturing of C&GT products is very complex and they have to be released in a short timeframe. These are high value products, available in limited quantity that should be controlled employing complex set of tests in order to ensure identity, safety and potency.

The current microbiological compendial methods like sterility and mycoplasma testing are not adapted to these products. During this tutorial industry experts will address the QC Testing Strategies to Improve Manufacturing Turnaround Time, the bioMérieux Microbiology Testing Solutions to Increase Operational Efficiency and Improve Patient Safety and will discuss How to Make Rapid Testing Mainstream for ATMPs through a Question & Answer session.

Refining Cell Therapy Logistics: Introducing the VIA Capsule
LOCATION: GLOBAL SHOWCASE THEATRE

Cathy Quirbach, PhD, Senior Product Manager – Cryopreservation and Cryogenic Cold Chain, Cytiva, United States
Alex Nancekievill, MBA, Chief Business Officer, Asymptote part of Cytiva, United Kingdom
Alex Guite, PhD, Vice President, Services and Alliances, World Courier, United Kingdom

Cytiva, formerly GE Healthcare Life Sciences, introduces the VIA Capsule, the first liquid nitrogen-free cryogenic shipment system, designed specifically for the transportation of cellular therapiess.

- Recognize the challenges experienced with cell therapy logistics today
- Understand how the VIA Capsule system delivers a more controlled, assured and patient focused way of shipping cellular products
- Discover how the VIA Capsule reduces the complexity, risks and inefficiencies associated with current shipping methods
- See the benefit of shipping the VIA Capsule within the World Courier global GDP certified network
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For more information talk with us at the Cytiva booth within the ISCT Exhibition Hall or visit Cytiva.com
Development and Clinical-scale Manufacturing of Potent CAR T Cell Therapies Using Transposon-Mediated Gene Transfer and Regulatory-compliant Electroporation

LOCATION: GLOBAL SHOWCASE THEATRE

Chairs: Lesley Eschinger, Director of Global Marketing, MaxCyte, United States
      Peter Gee, PhD, Field Application Scientist, Asia Pacific, MaxCyte, Japan

Currently, the gold standard in the manufacturing of chimeric antigen receptor T-cells (CAR-T) is based on viral gene transfer. As this is associated with high costs and safety regulations, it has been critical to develop a new viral-free CAR-T manufacturing protocol. This session will focus on the manufacturing of CAR-T cells by both piggyBac and Sleeping Beauty (SB) transposon gene delivery systems using optimized electroporation protocols for clinical applications. The viral free manufacturing process is designed in such a way that permits scale-up and reduction in the regulatory burden associated with conventional viral gene-transfer. Using an optimized, up-scaled protocol allows for the production of a CAR-T product at a magnitude that will permit treatment of patients, while showing high viability as well as antigen dependent tumor recognition and elimination in vitro. Experiments to determine the anti-tumor potency of the drug product in vivo and detailed genomic analyses are ongoing.

PART I: PIGGYBAC TRANSPOSON-MEDIATED CAR-T CELLS – A PROMISING AND REALISTIC APPROACH FOR CLINICAL APPLICATION

Shigeki Yagyu, MD, PhD, Kyoto Prefectural University of Medicine, Japan

PART II: CLINICAL-GRADE MANUFACTURING OF CAR T CELLS USING A NOVEL VIRUS-FREE PROTOCOL

Katrin Mestermann, PhD, Universitätsklinikum Würzburg, Germany

Implementing Rapid Microbiological Methods for Cell and Gene Therapy

LOCATION: GLOBAL SHOWCASE THEATRE

Chair: Evonne Fearnot, MSBME, MBA, Roche Custom Biotech, United States

Rapid microbiological methods (RMMs) are essential for state-of-the-art manufacturing efficiency of cell and gene therapies because traditional testing methods do not provide shorter testing timelines and lower sample volume utilization. Some cell and gene 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. Uncertainty around supplier selection, method validation, and costs associated with RMM implementation impede cell and gene therapy manufacturers from adopting these RMMs for QC testing. This session will provide real-world insight on the related issues around implementing a rapid mycoplasma PCR QC test in cell and gene therapy manufacturing to help push innovative rapid microbiological methods forward in a field that uniquely needs them.

USING ROCHE MYCOTOOL QPCR ASSAY FOR ATMP RELEASE OF CAR-T CELL PRODUCT APPROVED FOR CLINICAL STUDIES BY FDA, FAMPH AND MHRA

Sarah Snykers, PhD, Celyad, Belgium

ABBREVIATED IN-HOUSE QUALIFICATION OF TWO COMMERCIALY AVAILABLE MYCOPLASMA DETECTION KITS

Sowmya Viswanathan, PhD, University Health Network & University of Toronto, Canada
Driving Flexibility and Automation in Cell Therapy Manufacturing

**LOCATION: GLOBAL SHOWCASE THEATRE**

**Chair:** Carson Rhodes, MBA, Senior Manager, Global Marketing, Terumo BCT, United States

**Speakers:**
- Dalip Sethi, PhD, Senior Scientist, Scientific Affairs, Terumo BCT, United States
- Mark Jones, MS, Laboratory Scientist, Scientific Affairs, Terumo BCT, United States
- Nathan Frank, MS, Laboratory Scientist, Scientific Affairs, Terumo BCT, United States

To build a robust and automated process, it’s good to have a flexible and modular design that can adjust and expand to meet your demands as you move towards later phase clinical trials and ultimately commercialization. The Quantum Cell Expansion System can help take your research to the next level, regardless of the cell source you choose. Today, we will walk thorough three case studies showcasing how the Quantum flexibility can help further your research, while building a scaleable, robust and automated process.

- **CD3+ T Cells:** Clinical dose reached in 8-9 days, with consistent viability, fold expansion and doubling times
- **Tregs:** Increase Scale and Viability with Automation
- **MSCs:** Culture up to 1.9 Billion MSCs in 5 days using Rooster Bio MSCs and xeno-free medium.
ON-DEMAND PRE-RECORDED GLOBAL SHOWCASE PRESENTATIONS (15 MINUTES)

Tune the Drivers of Immune Cell Potency, Fate and Fitness

LOCATION: GLOBAL SHOWCASE THEATRE

Ian Hayes, BSc (Hons), PhD, Pharma Market Manager, Cell Analysis Division, Agilent Technologies, Ireland

Immuno therapy is changing the landscape of cancer treatment, but most available tools are adapted and not purpose-built for this cell-centric workflow. Agilent Technologies is dedicated to supporting these next-generation therapies, providing key technologies to measure immune cell function, enabling researchers to achieve the necessary level of therapeutic potency and safety.

Scalable MSC Manufacturing in Three Bioreactor Systems Using hPL Supplements

LOCATION: GLOBAL SHOWCASE THEATRE

William Milligan, VP Business Development, AventaCell Biomedical Corp., Ltd., Taiwan
Cláudia Lobato da Silva, PhD, Associate Professor, iBB-Institute for Bioengineering and Biosciences, Instituto Superior Técnico, Universidade de Lisboa, Portugal

Large cell doses (>1x10^6 cells/kg) have been required for clinical implementation of MSC-based therapies and the success in obtaining those cell numbers starting from select human tissues is dependent on efficient ex-vivo expansion protocols able to comply with GMP. In past years, we have established scalable expansion of human MSC in bioreactors and have demonstrated potential to maximize cell productivity by changing several parameters, including the utilization of serum-/xenogeneic-free (S/XF) culture supplements, as well as different tissue sources, and bioreactor configurations. This talk will summarize methods and primary results for scalable hMSC expansion in three bioreactor systems using human Platelet Lysate (hPL) supplements (UltraGRO™ brands):

1. Microcarrier-based spinner flask system for the expansion of umbilical cord (UC)-derived MSC.
2. Terumo’s hollow fiber Quantum Cell Expansion System for the expansion of adipose tissue (AT)-derived MSC.
3. Microcarrier-based PBS Vertical Wheel™ Bioreactor for the expansion of both UC- and AT-derived MSC.

Our focus was to establish an integrated platform using hPL supplements capable of supporting efficient isolation (from primary tissue samples) and cost-effective expansion of hMSC for clinical development and commercial scale production.

DURA Innovations Dry Reagent Technology for Rigorous Phenotyping of Human Mesenchymal and Hematopoietic Stem Cells

LOCATION: GLOBAL SHOWCASE THEATRE

Michael Kapinsky, PhD, Senior Marketing Manager, Beckman Coulter Life Sciences, Germany

While there is solid consensus on the crucial phenotypic features associated with multipotent human mesenchymal (Dominici et al. Cytotherapy 2006) and hematopoietic stem cells (Dmytras et al. BM Transplant 2016; Cimato et al. 2019), substantial bias and lack of precision continue to concern flow cytometry users. A major portion of the variability observed can be contributed through sample preparation by human operators. The DURA Innovations dry reagent technology eliminates manual antibody pipetting, provides expert antibody panel design and assures lot-to-lot consistency, supporting the scientific rigor needed in clinical stem cell research.
How 30+ years of Experience Has Powered Business Continuity During the COVID-19 Pandemic

LOCATION: GLOBAL SHOWCASE THEATRE

Julie Smolich, MBA, Senior Vice President, Provider Services, Be The Match BioTherapies, United States

The COVID-19 pandemic has brought new challenges to the already complex delivery of cell and gene therapies. Now, more than ever, it’s critical that your cell therapy supply chain is expertly designed, managed, and utilizes infrastructure that’s proven to deliver through even the most unprecedented times. Throughout the past 30+ years, our expertise and established relationships have successfully delivered 100,000+ time-sensitive cell therapies. In this presentation, you’ll receive expert insight from Be The Match BioTherapies about the systems, teams and infrastructure that’s critical to minimizing variability throughout the cell therapy supply chain and ensuring patients receive the therapy their life depends on. Learn more: https://bethematchbiotherapies.com

Cryopreservation of hMSC for Cell Therapy
A Novel Defined 5% DMSO Salt-Based Freezing Solution

LOCATION: GLOBAL SHOWCASE THEATRE

Mira Genser-Nir, PhD, R&D Project Manager, Biological Industries, Israel

hMSC are a critical raw material for cell-based therapies and usually a large number of cells are required for clinical applications.

Cryopreservation is currently the only method to preserve cells with maintained functional properties and genetic stability for long term storage.

To date, a common practice is use of homebrew cryo-medium that is composed of animal-derived raw materials and low Mw Cryoprotective agents (CPAs), usually 10-20% DMSO. Alternatively, animal component free (ACF) freezing solutions were developed for clinical applications, but they are still based on culture medium and are composed of 10% DMSO (e.g. NutriFreez™ D10). In addition, a few products were developed with a reduced concentration of DMSO or even without DMSO. However, these products appear not to be optimal for hMSC. Moreover, most of the commercially available DMSO-free products are actually composed of other potentially toxic permeable CPAs (e.g. Ethylene Glycol, EG). Exposure of cells to these materials can impact the quality, safety and efficacy of cell-based therapeutic product. Facing strict regulatory requirements, the development of defined, ACF, salt base freezing solution with reduced concentration of DMSO is essential.

The current study presents the feasibility of a novel defined, ACF, protein free, salt base freezing solution composed of 5% DMSO for cryopreservation of hMSC from various sources. Results show that the novel cryopreservation solution efficiently maintains high cell yield and viability and supports the recovery of hMSC while maintaining the normal hMSC features: typical fibroblast-like cell morphology, phenotypic surface marker profile, differentiation capability as well as self-renewal potential.

Clinically accepted, cryopreservation solution for hMSC holds a unique opportunity to facilitate the translation of these cells to cellular therapy applications.
Mesenchymal Cell Therapy for Covid-19: Is Repurposing Enough?

**LOCATION: GLOBAL SHOWCASE THEATRE**

**Tomer Bronshtein, PhD, Research Manager, Bonus Therapeutics Ltd., Israel**

With the alarming spread of SARS-CoV-2, several companies have started testing repurposed MSC-based therapies for Covid-19 patients suffering from acute respiratory distress (ARD). These efforts are partially supported by prior studies that evaluated MSCs in inflammatory lung diseases, and which produced compelling safety data, albeit providing no evidence for clinical efficacy. Caution is advised, therefore, against the use of repurposed treatments in Covid-19 patients, especially in light of recent data showing marginal benefits of repurposed drugs in an increasing number of patients, despite high initial expectations.

With this warning in mind and relying on years of MSC-related experience, in-house MSC manufacturing capacity, and the technologies, we developed, for the efficient and standardized isolation and cultivation of MSCs, Bonus Therapeutics has developed MesenCure, an enhanced allogeneic MSC-based product designed, explicitly, for treating ARD in Covid-19 patients.

Preclinical results show that MesenCure, enhanced by biological, chemical, and physical means, but not untreated MSCs, have managed to alleviate lung edema and reduce lymphocytes’ infiltration. These results imply that naïve MSCs, merely repurposed, might not be enough, as well as emphasize the prospects of MesenCure in Covid-19. Bonus Therapeutics is continuing the development of MesenCure and expects to enter clinical trials within six months.

**One Hour Mycoplasma Testing by Anyone, Anywhere, Anytime**

**LOCATION: GLOBAL SHOWCASE THEATRE**

**Sylvanie Cassard Guilloux, PhD, Global Solution Manager, bioMérieux, France**

This innovative solution to test mycoplasma is a game changer, it allows a broad detection in one hour, is totally automated and really easy to use. This technology is based on a “lab in a pouch” disposable that contains all reagents and controls necessary for a rapid molecular test. Thanks to its automation from sample to result with 2 minutes of hands on time, and its low footprint, this solution can be used for at line testing outside of the context of a high level of expertise laboratory. This is the first method allowing at-line in-process controls, and rapid release of products in 1h.

**Commercial CAR-T Tech Transfer Considerations**

**LOCATION: GLOBAL SHOWCASE THEATRE**

**Dominic Wall, PhD, Associate Professor, Chief Scientific Officer, Cell Therapies Pty Ltd, Australia**

**Gerry McKiernan, Director of Quality, Cell Therapies Pty Ltd, Australia**

**Shae Disney, CAR-T Logistics Manager, Cell Therapies Pty Ltd, Australia**

Conducting the tech transfer of a commercial CAR-T product is a challenging proposition. There are many considerations that a manufacturing organization must undertake to successfully overcome these challenges. Our presentation will focus on the different aspects of a commercial CAR-T tech transfer – business, quality & regulatory, and clinical supply chain & logistics – that are critical to successful implementation.
May 29th, 2020
4:00 pm – 5:00 pm CEST
with Dr. Sebastian Warth

We are looking forward to meeting you at our virtual booth!

CellGenix® T Cell Medium

Learn more
Ask our Expert
Introducing CellGenix® T Cell Medium – A Serum- and Xeno-Free Medium For Improved T Cell Therapy Manufacturing

LOCATION: GLOBAL SHOWCASE THEATRE

Sebastian Warth, PhD, Senior Scientist, CellGenix GmbH, Germany

The manufacturing process of T cell products requires reagents that meet the regulatory guidelines and ensure cellular products of consistent quality. Many current manufacturing protocols rely on human serum-containing media. Human serum requires extensive testing prior to use for production of cellular products due to lot-to-lot inconsistencies. Moreover, human serum is a limited resource and might not be available in quantities needed for commercial manufacturing.

The CellGenix® T cell Medium (CellGenix® TCM) offers a ready-to-use, serum-free and xeno-free alternative for rapid expansion of functional human T cells. Cultures in CellGenix® TCM exhibited high cell numbers early after activation and throughout culture with high cell viability.

T cells expanded in CellGenix® TCM acquired an early-differentiated phenotype and a high proportion of polyfunctional cells. CAR T cells generated with CellGenix® TCM achieved a high “specific killing” of target cells and demonstrate the functionality of the T cell product. High expansion and viability with CellGenix® TCM in a classic culture dish translate well to the G-Rex® culture device with enhanced gas exchange, which is widely used in clinical settings.

EFS, ATMP CDMO Services

LOCATION: GLOBAL SHOWCASE THEATRE

Sophie Derenne, PharmD, PhD, National head of ATMPs and ABC Platform manager, EFS, France

Béatrice Araud, National Key account Manager, EFS, France

EFS is the only French National transfusion service. Moreover, EFS puts its experience in Research so as to facilitate access to new treatments, for cell and gene therapies.

EFS has become a key ATMP European player in the development and production of innovative therapies (CDMO) drugs.

EFS Pharmaceutical Establishment has 5 platforms, with 16 ATMP - Class B production rooms. EFS expertise covers a wide range of GMP services, from development up to clinical stages manufacturing, in the following areas:

- Immunotherapy (CAR-T Cells and Dendritic Cells)
- Regenerative Medicine (Mesenchymal Stem Cells - MSC, Differentiated Pluripotent Cells, iPS and hECS)
- Hematopoietic Stem Cells (CSH)
- Cell Bank Productions

EFS can support project owners (Academic, Biotech or Industrial) thru project diagnosis, process development, scale-up, GMP preclinical or clinical batches manufacturing, development and implementation of analytical tests (EP/ICH).

In addition, EFS can provide a strong support within a regulatory framework as well as a very helpful access to clinical staffs, promoting interface and advancement of clinical trials.

Some examples of successful collaborations: PDC Line Pharma /ADIPOA2 - European project H2020/ Clinical Trial Side by Cide/ Orthounion - Project H2020/ STREAM CECS ISTEM/ Protocol Excellent - Cellprothera.
Stimulating Growth. Cultivating Solutions
LOCATION: GLOBAL SHOWCASE THEATRE

Philipp Nold, PhD, Infield Application and Stem Cell Specialist, Eppendorf AG, Germany

Large cell numbers are needed for the development of cell therapies and stem cell-based drug research applications. By utilizing its strong synergies in cell culture expertise, bioreactor technology, and polymer manufacturing, Eppendorf has emerged as an expert partner for the cultivation of stem cells at large scale. With our equipment, training programs, and application services, we support scientists in resolving cultivation bottlenecks during the development of advanced stem cell-based applications. The need for advanced solutions is increasing. With our expertise, we help to stimulate the growth of your cultures and cultivate solutions tailored to your challenges.

Applying Acoustics to Cell Processing, an Evaluation
LOCATION: GLOBAL SHOWCASE THEATRE

John Zhao, MS, Senior Associate Scientist II, Cellular Process Development and Gene Editing, bluebird bio, United States

Cell wash, concentration and final formulation in the cell therapy field remains a challenge. Once cells are harvested, time and technology suitability, are critical to ensure processing conditions will not affect cell viability and activity. With the application of acoustic wave separation techniques, which enables a lower shear separation method, these critical process steps have a new solution. In this presentation, we will review the evaluation of the FASTBox device, the precursor to the ekko™ acoustic cell processing system, at bluebird bio, for effective volume reduction and media replacement for a CAR-T drug product.

Advancing the Development and Manufacturing of Cell and Gene Therapies and Building the Workforce of the Future
LOCATION: GLOBAL SHOWCASE THEATRE

Haro Hartounian, PhD, Senior Vice President & General Manager, Biopharma Division, New Jersey Innovation Institute, United States

There is a collaborative effort underway at the New Jersey Innovation Institute (NJII) to advance the biopharma industry. NJII is a non-profit owned by one of the top polytechnic universities in the United States and its Biopharma division and its advisors represent some of the most influential members of the industry. NJII has the only facility designed to provide flexible process development and clinical manufacturing of cell and gene therapies located on a University campus in the United States. In addition the team is pioneering several efforts related to workforce development and the support of innovative biopharmaceutical companies focusing on cell and gene therapies.

Dr. Haro Hartounian, a 30 year expert and the general manager of the division, will lead this presentation describing the types of collaborative efforts and projects underway as well as the capabilities of BioCentriq, the division’s cell and gene therapy development center.
Transient Transfection at Large Scale for Clinical AAV9 Vector Manufacturing

Rachel Legmann, PhD, Director, Technical Consultancy - Gene Therapy and Viral Vectors, Pall Biotech, United States

Gene therapy clinical trials often require high titer vector preparations to adequately deliver the therapeutic transgene, in great excess of research-level production utilized in many laboratories. Bioprocessing for the therapeutic agents for these therapies still face many challenges during scale up. Here, we will present a case study which illustrates the challenges and solutions to scale up process steps required to manufacture AAV9.

We evaluated AAV9 production in adherent HEK293 cells utilizing the small scale iCELLis Nano fixed-bed benchtop bioreactor. Based on conditions developed in the pilot iCELLis Nano bioreactor system, we scaled this production to the large scale iCELLis 500 bioreactor (200 m² and 333 m² surface area). A HEK293 cell seed train was utilized, using the Xpansion® 200 bioreactor to generate sufficient cell numbers for seeding the bioreactor. Transfection reagents were scaled for efficient transfections. Media components, such as glucose as well as O₂ and CO₂ were evaluated and replenished as needed throughout the run using the perfusion process of the iCELLis bioreactor. Following a production phase, >10⁶ vector genomes were isolated from crude harvest of the bioreactor.

Scalable, Single-Use Technologies for Production and Purification of Viral Vectors

Todd Sanderson, BS, Senior Manager R&D, Pall Biotech, United States

Recent advances in cell and gene therapies have opened the door to bringing life-saving, curative treatments to many patients and families. Many of these therapies rely on adeno-associated virus (AAV) or lentivirus (LV) viral vectors for gene transfer. Industrialization of these therapies requires robust, scalable manufacturing processes. Existing technologies can be readily adapted for viral vectors. Planar and fixed-bed adherent bioreactors are valuable tools in upstream vector production for both seed train biomass and vector production. Purification of these vectors can be achieved using scalable, single-use technologies including direct-flow filtration (depth and sterile), membrane-based ion exchange chromatography, and tangential-flow filtration (TFF). Here we present a strategy for utilizing these single-use technologies from Pall Biotech for both AAV and LV manufacturing platforms.

Using Vertical-Wheel Bioreactors to Overcome Bioprocess Challenges in Expansion of hiPSC Aggregates from Single Cells

Breanna Borys, BSc, Senior PhD Candidate, University of Calgary, Canada

Human induced pluripotent stem cells (hiPSCs) hold enormous promise in accelerating breakthroughs in understanding human development, drug screening, disease modeling and cell and gene therapies. Their potential, however, has been bottlenecked in a mostly laboratory setting due to bioprocess challenges in the scale-up of large quantities of high-quality cells for clinical and manufacturing purposes. While several studies have investigated the production of hiPSCs in bioreactors, the use of conventional horizontal-impeller, paddle and rocking-wave mixing mechanisms have demonstrated unfavourable hydrodynamic environments for hiPSC growth and quality maintenance. We developed a scalable, single-cell inoculation protocol which successfully maintained cell growth rates without sacrificing cell quality, and we have provided the first published protocol for in-vessel hiPSC aggregate harvesting, permitting the entire bioreactor volume to be dissociated into single-cells for serial passaging into larger scale reactors. Importantly, the cells harvested and re-inoculated into scaled-up vertical-wheel bioreactors not only...
maintained consistent growth kinetics, they maintained a normal karyotype and pluripotent characterization and function. We have demonstrated the success of these protocols in cultivating large quantities of hiPSC aggregates in vertical-wheel bioreactors using a wide variety of commercially available pluripotent stem cell media types.

Welcome to Cellicon Valley: Philadelphia, Pennsylvania, USA

LOCATION: GLOBAL SHOWCASE THEATRE

Jonathan A. Epstein, MD, Executive Vice Dean and Chief Scientific Officer, William Wikoff Smith Professor of Medicine, Perelman School of Medicine at the University of Pennsylvania, United States

In Cell and Gene therapy, the race is on to optimize manufacturing, advance technical capabilities and assemble the necessary teams to broaden application and impact. Philadelphia and Penn Medicine are leading national and global efforts in Cell and Gene Therapy and Connected Health – Welcome to Cellicon Valley.

Next-generation Transfection Reagent for Large Scale rAAV Manufacturing

LOCATION: GLOBAL SHOWCASE THEATRE

Mathieu Porte, PhD, R&D Manager Bioproduction, Polyplus-transfection, France

The number of ATMP therapeutic-based medicines for inherited genetic disorders is in constant growth, with a global 32% increase in new clinical trials in the last 4 years. ATMPs have demonstrated their success with already more than ten approved for commercialization. The success of AAV as the most promising viral vector for gene therapy is due to low immunogenicity, broad tropism and non-integrating properties. One major challenge for translation of promising research to clinical development is the manufacture of sufficient quantities of AAV. Transient transfection of suspension cells is the most commonly used production platform, as it offers significant flexibility for cell and gene therapy development. However, this method shows some limitations in large scale bioreactors: inadequate transfection protocol, reduced transfection efficiency and lower productivity. To address this concern, we present data on the novel transfection reagent showing: i) increased AAV titers, ii) improved transfection protocol for large scale bioreactors and iii) reproducibility of viral titers at different production scale. The aforementioned optimized parameters make this novel transfection reagent ideal for cell and gene therapy developers by combining the flexibility of transient transfection with scalability and speed to market.

Rapid Scale-up and Clinical Translation of hMSCs and hMSC-EVs

LOCATION: GLOBAL SHOWCASE THEATRE

Xuan Xu, PhD, Field Application Scientist, RoosterBio, United States

Human Mesenchymal Stem/Stromal Cells (hMSCs) are critical raw materials in numerous therapeutic approaches including cell therapies, cell-based gene therapies, tissue and organ engineering, medical devices and exosome and extracellular vesicle-based therapies. RoosterBio, Inc. is a privately held cell manufacturing platform technology company focused on accelerating the development of a sustainable Regenerative Medicine industry, one customer at a time. RoosterBio’s high-volume, cost-effective and well-characterized adult hMSCs paired with highly engineered bioprocess media systems are built for rapid manufacturing scale-up, thus removing several years and millions of dollars from product development and clinical testing. This approach revolutionizes how regenerative medicine products are developed, clinically translated, and commercialized. In response to the current COVID-19 pandemic, our scalable hMSC manufacturing systems could address therapeutic needs for a huge demand of clinical grade hMSCs and hMSC-derived products. The newly launched product RoosterCollect™-EV-CC is the world’s first cGMP extracellular vesicle collection medium. This product is a key addition to RoosterBio’s cGMP solutions.
Customized Medical Disposables

- Tubing Sets
- Filtration
- Spikes

- R & D
- Production
- Regulators
- Injection Moulding
- Assembly

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Agilent is a leader in life sciences, diagnostics and applied chemical markets. The company provides laboratories worldwide with instruments, services, consumables, applications and expertise, enabling customers to gain the insights they seek. Agilent’s expertise and trusted collaboration give them the highest confidence in our solutions.

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.

Aseptic Technologies helps biopharma companies to deliver Cell and Gene Therapy Products to their patients by bringing innovation to the aseptic filling operation. Used for the cGMP manufacturing of individualized, off-the-shelf therapies and intermediate products since 2009, the AT-Closed Vial® Technology is a leading aseptic filling solution. The central element of the technology is the unique vial, remaining closed throughout the filling process and ensuring 100% Container Closure Integrity during -80°C and cryogenic storage.

The benefits of the AT-Closed Vial® Technology:

- Safe -80°C/cryogenic storage, no fragilization, better sterility assurance, reduced COGs, fast manual closed operations, scaling-up with robotized process.

NEW! Visit our virtual booth to see Crystal® Pure M1: the first plug-and-play isolated aseptic filling unit, dedicated to safer cost-effective small batch operation.

The American Society for Transplantation and Cellular Therapy is an international professional membership association of more than 2,200 physicians, investigators and other health care professionals from more than 45 countries. Our mission is dedicated to improving the application and success of bone and marrow transplantation and related cellular therapies. We strive to be the leading organization promoting research, education and clinical practice in the field.

Avectas is a cell engineering technology business focused on improving the cost, manufacturing and patient outcomes for the next generation of cellular therapies.

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 a range of human-derived cell culture supplements for use in translational research and development of cell and tissue-based therapies. AventaCell launched gamma irradiated UltraGRO™ products in 2018, to meet the demand of regulatory
authorities for pathogen reduction treatment for hPL / FD hPL supplements to support end users in advancing cell therapies to clinical development and commercialization. The Company is also launching UltraKURE™-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 UltraGRO™ supplements in various 3-D bioreactor systems (e.g. micro-carrier spinner flasks, Terumo's Quantum System and PBS' vertical wheel system) for MSC production. The demand for safe, efficient, cost-effective cell expansion and production is rapidly increasing with 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.

Be The Match BioTherapies is the only cell and gene therapy solutions provider with customizable services to support any stage of the supply chain, from the point of patient identification through cell harvest, therapeutic intervention and long-term outcomes data collection. Backed by the unrivaled experience of the National Marrow Donor Program®/Be The Match®, and in collaboration with the CIBMTR® (Center for International Blood and Marrow Transplant Research®), our experts design solutions that advance cell and gene therapies in any stage of development — from research through commercialization.

Berkeley Lights, Inc. develops and commercializes workflows to find the best cells. By operating at the intersection of biology, technology and information, our workflows accelerate the design, discovery, development, and delivery of cell-based products enabling the deployment of biology for the production of sustainable sources of food, therapies, and energy.

BioCanRx is Canada's Immunotherapy Network. Our vision is to cure patients and enhance the quality of life of those living with cancer. We invest in leading edge immune oncology research translating world-class technologies from the lab into early phase clinical trials. BioCanRx provides researchers with access to funding, expertise, training and manufacturing facilities and is a leader in the translation, manufacture and adoption of cancer immunotherapies.

BioLife Solutions is a leading supplier of cell and gene therapy bioproduction tools. Our tools portfolio includes our proprietary CryoStor® freeze media and HypoThermosol® shipping and storage media, ThawSTAR® family of automated, water-free thawing products, evo® cold chain management system, and Custom BioGenic Systems high capacity storage freezers.
Founded in 1981, Biological Industries specializes in the development, manufacture, and distribution of products for cell culture, focusing today on stem cell systems and media for cell therapy.

BI offers a full range of xeno-free stem cell products and services, which includes stem cell culture media, freezing media, attachment factors, and cell dissociation solutions.

BI products include the Nutristem® range of cell media (for mesenchymal, induced pluripotent, and embryonic stem cells), and BIOTARGET (for T cells), all serum-free, xeno-free media for research and clinical applications, helping to advance stem-cell-based therapies.

We are committed to a Culture of Excellence through our advanced manufacturing and quality-control systems, superior regulatory expertise, and extensive technical customer support, training, and R&D capabilities.

BlueRock is using its unique cell+gene platform to direct cellular differentiation and genetically engineer cells to create an entirely new generation of cellular medicines in the areas of neurology, cardiology, and immunology.

Cryogenic storage is critical to maintain chain of identity and condition for cell-based materials. Brooks Life Sciences delivers scalable cryogenic infrastructure that strengthens your quality and documentation processes.

C3i provides an integrated structure to accelerate the discovery, development, commercialization and access to innovative cancer immunotherapies and regenerative medicine. One of the biounits represented by C3i is the Centre of Excellence for Cellular Therapy (CETC), a fully operational state-of-the-art cGMP manufacturing facility for cellular therapies, including cancer immunotherapies.

Located within the Hospital Maisonneuve-Rosemont in Montreal, the CETC facilities have been validated and met expectations for the Canadian, European Medicines Agency (EMA) and U.S. Food and Drug Administration (FDA) regulatory agencies. The CETC has received the highest certification from the U.S. Foundation for the Accreditation of Cell Therapies (FACT) for “more than minimally manipulated cells” and is the largest operational Good Manufacturing Practices (GMP)-validated centre in Canada.
The Cell and Gene Therapy Catapult was established as an independent centre of excellence to advance the growth of the UK cell and gene therapy industry, by bridging the gap between scientific research and full-scale commercialisation. With more than 180 employees focusing on cell and gene therapy technologies, it works with partners in academia and industry to ensure these life-changing therapies can be developed for use in health services throughout the world. It offers leading-edge capability, technology and innovation to enable companies to take products into clinical trials and provide clinical, process development, manufacturing, regulatory, health economics and market access expertise. Its aim is to make the UK the most compelling and logical choice for UK and international partners to develop and commercialise these advanced therapies. The Cell and Gene Therapy Catapult works with Innovate UK.

For more information please visit ct.catapult.org.uk or visit http://www.gov.uk/innovate-uk.

Cell Therapies Pty Ltd is a contract development and manufacturing organization (CDMO) that manufactures and deploys advanced cell-based therapies to the global market. Established in 2003, Cell Therapies Pty Ltd is one of the most experienced cGMP compliant manufacturers for cell therapies, gene therapies, cellular immunotherapies and regenerative medicine products globally. Working closely with its clinical and research collaborators at the Peter MacCallum Cancer Centre (http://petermac.org), Cell Therapies Pty Ltd provides its clients with needle-to-needle solutions for apheresis collection, GMP manufacturing, cryopreservation, state of the art imaging (real-time in vivo cell tracking) and clinical trial implementation.

Cell Therapies Pty Ltd has a ten (10) clean room GMP facility located in Melbourne, Australia at the heart of the biomedical precinct, with access to hospitals, research institutes and universities to support development, translation and patient access. This facility holds both clinical trial and commercial supply manufacturing licenses from the Australian Therapeutic Goods Administration (TGA), ensuring the products from our facility meet global regulatory standards.

We have a proven track record of delivering cell-based therapeutic products to patients from early phase clinical trials through to commercial supply according to the requirements of local and international regulators. We have also established multi continental manufacturing networks to support trials concurrently registered in Europe, Asia and North America and have an enviable track record for efficient and successful tech transfer inbound and outbound to our collaborators.

Please contact us through our website (http://www.celltherapies.com.au) to discuss potential collaborations.

CCRM is a Canadian, not-for-profit funded by the Government of Canada, the Province of Ontario, and leading academic and industry partners. It supports the development of regenerative medicines and associated enabling technologies, with a specific focus on cell and gene therapy. CCRM has a 40,000 square foot space dedicated to advanced cell manufacturing that includes a fully resourced development facility used to both evaluate and advance technologies.

CCRM aims to accelerate the translation of scientific discovery into new companies and marketable products for patients. CCRM is the commercialization partner of OIRM and the University of Toronto’s Medicine by Design.
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 improve the 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.

CellGenix is a leading global supplier of high quality raw materials for the expanding market of cell and gene therapy and regenerative medicine. CellGenix develops, manufactures and markets human cytokines, growth factors, and other recombinant cell culture components in preclinical and GMP quality as well as proprietary serum-free media for further manufacturing of ATMPs. CellGenix products are used by academia and industry partners in clinical trials and commercial manufacturing throughout the world. As an ATMP developer and manufacturer, CellGenix gained in-depth cell culture knowledge and superior regulatory expertise. With this unique background, CellGenix understands the high requirements their customers face during product development and the regulatory approval process. To meet the increasing demand for GMP quality raw materials for ATMP manufacturing CellGenix has recently expanded its manufacturing capacity and has built additional R&D and QC laboratories, and warehouse space. The upgrade also introduced state-of-the-art, automated, large-scale capacities for recombinant protein products in the existing GMP facilities. CellGenix is headquartered in Freiburg, Germany and operates a subsidiary near Boston in Portsmouth, USA. https://cellgenix.com/

The Cord Blood Association is an international, non-profit organization that promotes both public and family cord blood banking, with the objectives of saving lives, improving health and changing medicine.

The association’s priorities are advocacy, quality products and services, market expansion, research and development, and public and professional education. Members of CBA include both public and family banks and individuals in and served by the cord blood community including cord blood bank personnel, research investigators, laboratory technicians, patients, donors, regulatory officials, vendors and health care providers such as transplant physicians, obstetricians, pediatricians, nurses and midwives.

STEM CELLS Translational Medicine is the association’s official scientific journal. More information about the CBA can be found on its website at www.cb-association.org.

Corning Life Sciences’ line of advanced cell culture surfaces, scalable vessel platforms, and cell culture media provide innovative solutions for stem cell research. Products include the established Matrigel® Matrix, novel animal-free surfaces for defined stem cell expansion, and media for increased expansion of hMSCs.

Corning offers an industry-leading line of advanced surface, media, and scalable platforms for cell processing or cell therapy indications. Corning’s family cell culture platforms includes flasks, roller bottles, CellSTACK® and HYPERStack® vessels, and the CellCube® perfusion system, as well as ready-to-use microcarriers. Corning also provides scalable cell culture environments for stem and primary cell types.
with clinical potential, including an extensive line of both vialled and pre-coated extracellular matrices such as Matrigel® matrix and defined animal-free ECM mimetic substrates and surfaces, like Corning PureCoat™ laminin-521 cultureware. Corning media solutions include standard and custom cGMP media, as well as basal salt solutions, antibiotics, sera, and flexible packaging solutions. For more information, visit www.corning.com/lifesciences

Cytiva is a global provider of technologies and services that help advance and accelerate the development and manufacture of therapeutics. Previously GE Healthcare Life Sciences, Cytiva’s diverse portfolio includes well-recognized brands such as ÄKTA, Amersham, Biacore, FlexFactory, HyClone, MabSelect, Sefia, Whatman, Xcellerex and Xuri. Cytiva brings speed, efficiency and capacity to research and manufacturing workflows, enabling the delivery of transformative medicines to patients. Visit cytivalifesciences.com for more.

EFS, a European ATMP CDMO offering a full range of services : ATMP production (C&GT), process development & scale-up services, and quality controls

Visit our booth and learn about our bioprocess solutions for stem cell cultivation. Get in touch with our experts to find the fitting solution for your process or check our interesting information material. We are looking forward to meeting you online!

Esco Aster is a contract development & manufacturing organization (CDMO) focused on offering vaccine-, bio- cell & gene-therapy development manufacturing services using primarily its proprietary Adherent Tide Motion Platform. Visit www.escoaster.com for our latest updates.

FACT establishes standards for high quality medical and laboratory practice in cellular therapies. FACT is a non-profit corporation co-founded by the International Society for Cellular Therapy (ISCT) and the American Society for Transplantation and Cellular Therapy (ASTCT) for the purposes of voluntary inspection and accreditation in the field of cellular therapy.
Fresenius Kabi is a global healthcare company that specializes in lifesaving medicines and technologies for infusion, transfusion and clinical nutrition. We bring over 60 years of experience advancing the fields of cell collection and separation with expertise in closed-system processing. Our Lovo Cell Processing System is the only cell processing system that washes and concentrates white blood cells using filtration technology, specifically to serve the needs of the cellular therapies community.

FUJIFILM Irvine Scientific

FUJIFILM Irvine Scientific, 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, FUJIFILM Irvine Scientific supplies the cytogenetic, immunotherapy, biopharmaceutical, cell and gene therapy, and Assisted Reproductive Technologies (ART) industries with a range of advanced cell culture media products and expert development, optimization, and commercial manufacturing services.

Genezen Laboratories

Genezen offers process development, viral vector manufacturing, and transduced cell manufacturing. We offer expertise and flexible GMP capacity to expedite your development timelines in our facilities and through our master service agreements with leading research institutions.

Harro Höfliger specializes in the development of customer-oriented process and production solutions for aseptic, pharmaceutical, and medical device applications products. Our core expertise with innovative machine platforms is in customized sterile automation solutions for product assembly, processing of web materials, as well as dosing and inhalation technology.

The portfolio of upscalable test machines and modules, as well as technology platforms to meet your precise requirements, comes from many years of experience in targeted research and development. Therefore, Harro Höfliger covers all phases of your project from the laboratory to high efficiency production.

The Health and Environmental Sciences Institute (HESI) is a non-profit institution whose mission is to collaboratively identify and help resolve global health and environmental challenges through the engagement of scientists from academia, government, industry, NGOs, and other strategic partners. Since 1989, HESI has provided the framework for scientists from public and private sectors to meaningfully collaborate in developing science for a safer, more sustainable world.

HESI committees generate impactful collaborative science via a variety of mechanisms, including designing and conducting novel laboratory research, pooling and analyzing existing data, creating decision frameworks and methodologies, and identifying scientific best practices.

HESI’s Cell Therapy - TRAcking, Circulation, & Safety (CT-TRACS) committee was launched in 2016 to identify key needs for assessing the safety of cell therapies and identify opportunities to meet these needs. This program provides a neutral platform for cell therapy developers, researchers, regulators, imaging specialists, CROs, enabling tools developers and other
stakeholders to interact, discuss current challenges and identify best practices to ensure that these therapies are safe and effective for use. It brings together an international and multi-disciplinary team of experts with interest in sharing their knowledge, common challenges and seek consensus on finding harmonized solutions. In particular, the committee aims to bring awareness on how the application of existing cell tracking technologies, methods, and best practices can benefit the clinical translation of these new therapies. HESI is based in Washington, DC, USA but operates globally. Learn more about HESI’s scientific portfolio at hesiglobal.org.

ICCBBA is the not-for-profit, nongovernmental international standards 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.

IntelliStem is at the leading edge of a big leap in cancer treatment and was founded with the vision to revolutionize stem cell and immunotherapy development by harnessing the potentials for multipotent adult stem cells to overcome the limitations of traditional cancer treatments. IntelliStem’s enabling technology allowed the development of three platforms; Cancer Therapeutics Platform - To develop multiple therapies against cancer, Drug Discovery Platform (Intellipeptidome™) - To identify new targets, neoantigens and collaborate with other companies and platforms to develop monoclonal antibodies and CAR’s against cancers, Infectious Diseases Platform - To develop vaccines against infections without available therapies and drug resistant infections.

Lonza
Pharma & Biotech
Lonza is one of the world’s leading suppliers to the pharmaceutical, biotech and specialty ingredients markets. We harness science and technology to create products that support safer and healthier living and enhance the quality of life.

Macopharma creates quality innovative blood products and bio-sourced therapies for patients. We are dedicated to raising the standards of care by relying on close relationships with our suppliers and users, and questioning ourselves constantly to become the world’s most trusted healthcare partner.

Maintaining confidence by committing to a comprehensive business continuity plan and a sustainable development while fighting against all forms of discrimination are part of our values. We also believe that working collaboratively is a key element to meet challenges and imagine tomorrow’s products and services.

As yesterday and today, we will continue to support life in the future.

Established in 1984, MAK-SYSTEM designs, develops, and delivers globally best-of-breed software to orchestrate Cell and Gene Therapy. Our T.C.S. software provides unrivaled functionality, scalability, and technology to support all treatments across the complete supply chain thanks to the configurability of our software. It continuously supports the digital transformation of organizations such
as Tissue Banks, Stem Cell Labs, Cell and Gene Therapy (SME to Big Pharma, CMOs, Hospitals). Our experts are at the crossroad of healthcare and technology with an extended knowledge in your business to implement and support our software according to our clients’ needs.

MaxCyte is a global cell-based medicines and life sciences company applying its patented cell engineering technology to help patients with high unmet medical needs in a broad range of conditions. The company leverages its Flow Electroporation® Technology to enable its partners across the biopharmaceutical industry to advance the development of innovative medicines, particularly in cell therapy, including gene editing and immuno-oncology. MaxCyte has placed its cutting-edge flow electroporation instruments worldwide, including with nine of the top 10 global biopharmaceutical companies, and has more than 55 partnered program licenses in cell therapy including more than 25 licensed for clinical use. With its robust delivery technology, MaxCyte helps its partners to unlock the full potential of their products.

For more information, visit www.maxcyte.com.

Mill Creek Life Sciences is the first company to commercialize human platelet lysate for cellular therapy. A spin-off out of the Human Cellular Therapy Laboratory at the Mayo Clinic in Rochester, MN, Mill Creek has been involved in clinical cellular therapies from the beginning. Mill Creek is dedicated to providing the highest quality products for the research and clinical community.

With your need for rapid progress and vision for making a difference in the lives of patients, MilliporeSigma is your trusted partner in cell and gene therapy. Whether you are in preclinical development or commercializing your therapy, we provide a spectrum of products and services to help solve the challenges in cell and gene therapy manufacturing.

With over 20,000 employees and 72 manufacturing sites worldwide, MilliporeSigma’s portfolio spans more than 300,000 products enabling scientific discovery. The company is committed to solving the toughest problems in life science by collaborating with the global scientific community.

EMDMillipore.com/fastforward

Miltenyi Biotec develops cutting-edge solutions for biomedical research and cell and gene therapy applications. Our integrated cell manufacturing platform enables GMP-compliant cell processing and has been used in over 50,000 cell therapy procedures.

The New Jersey Innovation Institute (NJII) is an NJIT corporation focused on helping public and private organizations discover what’s possible. Whether it’s working to solve the grand challenges shared across an entire sector or helping a single company find an innovative way to pursue a new product or market opportunity, NJII brings world-class intellectual and technological resources to bear. We are unique in our formation and role as a not-for-profit corporation in pursuit of economic development and in our agility in transforming intellectual capital into commercial success.

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.

We strive to change the practice of medicine. We aspire to approach things differently—to make discoveries that take medicine in new directions. We look to tomorrow to inspire us today. Never satisfied with the status quo, we imagine what’s next.

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 manufactures the industry-preferred products for cell therapy. Our products include PermaLife Cell Culture Bag, CryoStore™ Freezing Bag, CryoPur DMSO Solutions and compatible Accessory Sets. Connect with us at OriGen.com.

PBS Biotech, Inc. is based in Camarillo, CA and manufactures the most advanced single-use bioreactors for the rapidly emerging cell and gene therapy market. Unique Vertical-Wheel™ mixing technology provides the benefits of homogeneous particle suspension with low power input, uniform distribution of turbulent energy dissipation rates, and minimal hydrodynamic shear stress. Compared to traditional stirred-type bioreactors with horizontal impellers, Vertical-Wheel bioreactors deliver superior performance for both the cell expansion and differentiation phases. As an example, for cells grown on microcarriers such as mesenchymal stem cells, Vertical-Wheel bioreactors facilitate high quality, scalable cell expansion as well as rapid, in-vessel medium exchange, cell dissociation from microcarriers, and harvesting. For cells grown as aggregates such as embryonic or induced pluripotent stem cells, Vertical-Wheel bioreactors allow for unmatched control of cell aggregate size and morphology while maintaining pluripotency, leading to highly efficient cell expansion and subsequent differentiation within bioreactors. With unparalleled scalability across a full range of vessel sizes, Vertical-Wheel bioreactors enable high-quality and efficient manufacturing of cell and gene therapy products, from research to clinical and even up to commercial scale. PBS Biotech also offers world-class contract research and process development services, with in-house laboratories and a dedicated Bioprocess R&D team that possesses industry-leading knowledge of a variety of cell and gene therapy applications.

Penn Biotech

Penn Medicine, part of world-renowned University of Pennsylvania, is at the forefront of cell and gene therapy. We discover treatments and cures that drive medical innovation, deliver state-of-the-art care and provide personalized medicine.
Supporting life science research since 1988, PeproTech is a privately owned biotechnology company focusing on the development and manufacture of high quality cytokine products for the life-science and cell therapy markets. Over the past 30 years the company has grown into a global enterprise with state-of-the-art manufacturing facilities in the US, and offices around the world.

Our mission is to provide the highest quality products that address the needs of today’s scientists and researchers, and we pride ourselves on being a trusted partner within the scientific community.

With over 2,000 products PeproTech has developed and refined innovative protocols to ensure quality, reliability and consistency.

Polyplus-transfection has been manufacturing and selling transfection reagents for over 15 years. Polyplus-transfection applies its expertise to the development of novel delivery solution for all types of nucleic acids (DNA, siRNA, miRNA...) for bioproduction (FectoPRO, PEIpro), research (jetCRISPR, jetPRIME, jetMESSENGER, jetPRIME) and therapeutics (in vivo-jetPEI).

Preserving Life For Tomorrow™
Protide Pharmaceuticals, Inc. is a fully integrated organization devoted to discovery, development and the advancement of technologies and processes in clinical cell therapy, regenerative medicine, transplantation and cell engineering.

With over 33 years of innovative products and services, and hundreds of clinical trials using our technology, we positively impact science through knowledge. Inspired by the scientists, patients, and physicians we support, Protide continues as an entrepreneurial, science driven organization.

Roche CustomBiotech offers high-quality solutions and documentation delivering real value in various cell and gene therapy manufacturing workflow steps from cell isolation to quality control release testing customizable to meet your needs.

RoosterBio is a privately held Maryland-based company focused on manufacturing and supplying human mesenchymal stem/stromal cells (hMSCs) that blast open the most significant bottleneck in clinical trial initiation and accelerate the regenerative medicine industry. Built for rapid scale-up, the company’s products are high-volume, efficient and well-characterized hMSCs paired with highly-engineered bioprocess media systems. RoosterBio has simplified and standardized how stem cells are purchased, expanded, and used in development, leading to marked time and costs savings for customers. RoosterBio’s innovative products are ushering in a new era of productivity and standardization into the field, accelerating the road to discovery in Regenerative Medicine.


Saint-Gobain Life Sciences develops and manufactures high-performance components and integrated solutions that touch a broad range of patient care, from the development of new therapeutic cancer treatments to biopharmaceutical production, on through to intravenous therapies for drug delivery.
To keep up with the rapidly evolving advanced therapeutics industry, you need an agile and experienced partner. Sartorius is a trusted global solution provider to the biologic industry offering a broad range of solutions from media and reagents to testing services and bioanalytics.

Scinus Cell Expansion BV, based in the Netherlands, develops and distributes bioreactor technology for stem cell cultivation.

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. The Scinus Cell Expansion™ system enables clinical scale cell 3D cultivation in a controlled environment and provides a cost effective alternative to 2D cell growth in tissue culture flasks or cell stacks.

The system contains a single use bioreactor bag that can be used to cultivate cells to clinically relevant numbers of cells.

The Tissue Engineering and Regenerative Medicine International Society (TERMIS) aims at worldwide development and application of science and technologies in tissue engineering and regenerative medicine. To accomplish this purpose, TERMIS brings together an international and interdisciplinary community of persons engaged or interested in the field and promotes education and research through regular meetings, publications and other forms of communication. This symposium is co-organized with the European Chapter of the Society (TERMIS-EU).

Terumo BCT believes in the potential of blood and cells to do even more for patients than they do today. Terumo BCT’s Cell Therapy Technologies business enables researchers, developers and manufacturers to create next-generation cell and gene therapies.
ThéCell is a non-profit provincial network supported by the Fonds de recherche du Québec – Santé (FRQS). Its mission is dedicated to further Cell, Gene and Tissue therapies. ThéCell mobilizes researchers and resources for the development of new therapeutic approaches in regenerative medicine. It aims to accelerate 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 120 researchers, clinicians and their teams (over 160 students and 50 research professionals) involved in key health research areas of cell, tissue and gene therapy, namely: 1) musculoskeletal and nervous systems; 2) cardiovascular, pulmonary, renal and digestive systems; 3) skin and cornea systems; and 4) hematology, oncology and immunology. Six infrastructures support the activities of the network including: preclinical, ethical and legal guidance, 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 within Canada and internationally.

World Marrow Donor Association (WMDA) is an association responsible for establishing consistent, high-quality standards for worldwide blood stem cell, marrow and cord blood unit donor registries/organisations. Worldwide, over 50,000 patients per year are in need of a matched donor outside their family. Nearly 50% of the patients with a match receive their blood stem cells, marrow or cord blood unit from a donor from another country. Therefore WMDA works towards a global standardisation by establishing an accreditation programme for registries. The accreditation programme ensures that organisations protect the welfare of the donors and provide high-quality blood stem cells for patients worldwide.

To learn more, visit: www.wmda.info.

Xcell Therapeutics successfully developed the First-in-Class chemically defined media, working with a team of talented and developing proliferation media for various cell types. It is our goal to become the CDMO leader in RM industry.

The Japanese Society for Regenerative Medicine (JSRM) is the largest society for regenerative medicine in the world, with over 6000 members involved in research in a wide variety of fields in the natural sciences. 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.
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 44 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 Satellite Sessions, Pre-Recorded Scientific Sessions and Quality and Operations Track Sessions from May 28-29, 2020.

In order to receive credit for this activity, participants must complete online evaluations for the sessions they attend.

Please visit www.isct2020.com to complete the evaluation form by June 30, 2020. CMLE certificates will be sent by email within 4-6 weeks of the program end date.

Learner Notification

ISCT 2020 Paris Virtual Meeting • May 28-29, 2020 Online

ACKNOWLEDGEMENT OF FINANCIAL COMMERCIAL SUPPORT

No financial commercial support was received for this educational activity.

ACKNOWLEDGEMENT OF IN-KIND COMMERCIAL SUPPORT

No in-kind commercial support was received for this educational activity.

SATISFACTORY COMPLETION

Learners must complete an evaluation form to receive a certificate of completion. Partial credit of individual sessions 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 19.50 AMA PRA Category 1 Credits™. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

OBJECTIVES – AFTER ATTENDING THIS PROGRAM YOU SHOULD BE ABLE TO:

1. Identify the scientific, clinical, laboratory, technology, funding and regulatory issues related to specific types of cell-based research/therapy.
2. Demonstrate cross-disciplinary participation from clinicians, laboratory personnel, regulatory professionals, scientists, technology and business experts from both academia and industry.
3. Describe the translation aspects of and issues involved with cell and tissue-based therapies.
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 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)

All individuals in a position to control the content of CE are listed in the program book. If their name is not listed below, they disclosed that they had no financial relationships with a commercial interest.

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<td>Elizabeth</td>
<td>Shpall</td>
<td>Novartis:Consultant, Magenta:Consultant, Adaptimmune:Consultant, Cellgene:Consultant, Partner Therapeutics:Consultant</td>
</tr>
<tr>
<td>Sandeep</td>
<td>Soni</td>
<td>Crispr Therapeutics:Consultant</td>
</tr>
<tr>
<td>Anthony</td>
<td>Ting</td>
<td>Athersys, Inc:Stock Shareholder</td>
</tr>
<tr>
<td>Sowmya</td>
<td>Viswanathan</td>
<td>Speaker Fees:Speaker’s Bursary for Roche for Mycoplasma Tutorial</td>
</tr>
<tr>
<td>Peter</td>
<td>Zandstra</td>
<td>Excellthera: Consultant, Excellthera: Stock Shareholder, Notch Therapeutics: Consultant, Notch Therapeutics: Stock Shareholder</td>
</tr>
</tbody>
</table>

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