Program

Scale-Up and Manufacturing of Cell-Based Therapies III

January 5 – 9, 2014
San Diego, CA, USA

Conference Chairs:

Chris Mason
University College London

Greg Russotti
Celgene Cellular Therapeutics

Peter Zandstra
University of Toronto

Engineering Conferences International
32 Broadway, Suite 314 - New York, NY 10004, USA
Phone: 1 - 212 - 514 - 6760, Fax: 1 - 212 - 514 - 6030
www.engconfintl.org – info@engconfintl.org
Hyatt Regency Mission Bay Spa and Marina - San Diego
1441 Quivira Road,
San Diego, California, USA 92109
Tel: +1 619 224 1234    Fax: +1 619 224 034
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Sunday, January 5, 2014

18:00 – 19:30  Conference check-in (Bayview Foyer)
19:30 – 20:30  Welcome Reception (Bayview Terrace)

NOTES

- Audiotaping, videotaping and photography of presentations are strictly prohibited.
- Please do not smoke at any conference functions.
- Turn your cellular telephones to vibrate or off during technical sessions.
- Technical sessions will be in Bayview I/II. Poster sessions will be in Bayview III.
- Meals will be in the Regatta Pavilion. The banquet on Wednesday will be in Mission II/III.
- Be sure to check your contact information on the Participant List in this program and make any corrections to your name/contact information online. A corrected copy will be sent to all participants after the conference.
- Speakers – Please leave at least 5 minutes for questions and discussion. Be available for discussion during meals and social periods.
Monday, January 6, 2014

08:00 – 09:30  Breakfast
09:00 – 09:45  Conference check-in (Bayview Foyer)
09:45 – 09:55  Welcome
Conference Chairs
ECI Liaison (Barry Buckland)
09:55 – 10:00  Introduction to Plenary 1
10:00 – 11:00  **Plenary 1**

**Stem Cells for Tissue and Organ Regeneration**
Mahendra Rao, National Institutes of Health, USA

**Session 1: Procurement, Handling and Processing of Tissue**
Chairs: Gary du Moulin (Genzyme)
Glyn Stacey (UK Stem Cell Bank, NIBSC)

11:00 – 11:25  Three decades of human tissue banking: Lessons learned for developers of cell therapy products
Silvia Chen (invited), LifeNet Health, USA

11:25 – 11:50  Material matters in cell therapy product development
Nicole Provost (invited), Biotechnology Consultant and USP BB2 Expert Committee Member, USA

11:50 – 12:10  Human-derived raw materials: Controlled, consistent collections enable successful manufacturing of cell-based regenerative medicine products
Thomas V. Ramos, HemaCare Corporation, USA

12:10 – 12:30  Ultra scale-down approach to membrane separation procedure of human cells for therapy; effect of cell concentration on cell loss
Maria Fernanda Masri, University College London, United Kingdom

12:30 – 14:00  Lunch

**Session 2: Shipping, Storage, Handling Product in Clinic and Delivery to Patient**
Chairs: Shelly Heimfeld (Fred Hutchinson Cancer Research Center)
Dolores Baksh (Organogenesis)

14:00 – 14:25  Implementation of cell- and gene therapy for clinical application: Impact of clinical requirements on the development
Volker Scherhammer (invited), Apceth GmbH & Co. KG

14:25 – 14:45  Multistem – Overcoming the logistical hurdles of a multi country trial
Ronald W. Fedechko (invited), Pfizer, USA

14:45 – 15:05  Expanded cord blood stem cells: would you like those fresh or frozen?
Ian Nicoud, Colleen Delaney (invited), Fred Hutchinson Cancer Research Center, USA

15:05 – 15:35  Coffee break
*Sponsored by Lonza Bioscience*
Monday, January 6, 2014 (continued)

15:35 – 15:40  Introduction to Plenary 2

15:40 – 16:30  **Plenary 2**
Raman spectroscopy to non-invasively monitor cell differentiation and nutrient limitation responses in culture
James Piret, University of British Columbia, Canada

16:30 – 18:10  **Poster Snapshots**

18:30 – 20:00  Dinner

20:00 – 22:00  **Poster Session and Social Hour** (with desserts)
Chairs: Peter Fuhrken (Cellular Dynamics International)
          Josh Leonard (Northwestern University)
Tuesday, January 7, 2014

07:30 – 09:00 Breakfast

**Session 3: Process Development Challenges for Allogeneic Products**
Chairs: Paula Alves (IBET)
        Ben Fryer (Betalogics)

09:00 – 09:25 *Learning from history and planning for the future - scale up of cell therapies for commercialization*
Anthony Davies (invited), Capricor

09:25 – 09:50 *Process development and scale-up of an allogeneic cell therapy product*
Koki Lilova (invited), Janssen Research & Development, Johnson and Johnson

09:50 – 10:10 *Robust cell manufacturing platforms integrated with novel proteomic and metabolomic tools to streamline the design of cardiac stem cell therapies*
Margarida Serra, ITQB-UNL/IBET, Portugal

10:10 – 10:30 *Metabolic consequences of defined media in pluripotent stem cell cultures*
Christian M. Metallo, University of California, San Diego, USA

10:30 – 10:50 *Scalable expansion of human induced pluripotent stem cells in xeno-free microcarriers*
Maria Margarida Diogo, Technical University of Lisbon, Portugal

10:50 – 11:20 Coffee break

11:20 – 11:25 Introduction to Plenary 3

11:25 – 12:25 **Plenary 3**
TBA
Gabor Forgacs, University of Missouri & Modern Meadow, USA

12:30 Boxed lunches available

13:00 – 14:20 **Session 4: Cell Therapy Manufacturing And Implementation Solutions**
Lunch Session: 4 talks - 20 min each

13:00 – 13:20 *Enabling allogeneic cell based product manufacturing transition from R&D to industrialization. Case study by Promethera Biosciences, a Cell Therapy Company*
Sarah Snykers, Promethera Biosciences, Belgium

13:20 – 13:40 *Cell processing facility with automated culture system based on the flexible modular platform*
Masahiro Kino-oka, Osaka University, Japan

13:40 – 14:00 *Scalable expansion and harvest solutions for allogeneic stem cells*
Daniel Kehoe, EMD Millipore Corporation, USA

14:00 – 14:20 *Development of a scalable manufacturing process for bone-marrow derived HMSC’s in a low-shear single-use bioreactor system*
Daniel Giroux, PBS Biotech, Inc., USA
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<td>Networking / Free time</td>
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<td>15:00 – 15:05</td>
<td>Introduction to Plenary 4</td>
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<td>15:05 – 15:55</td>
<td><strong>Plenary 4</strong>&lt;br&gt;Mark Post, Maastricht University &amp; Cultured Beef, The Netherlands</td>
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<td>15:55 – 16:20</td>
<td>TBA&lt;br&gt;Brian Hampson, Progenitor Cell Therapy, USA</td>
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<td>16:20 – 16:50</td>
<td>Coffee Break&lt;br&gt;<em>Sponsored by GE Healthcare</em></td>
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<td>16:50 – 17:15</td>
<td><strong>Process improvements for engineered T-cell manufacture to enable near-term commercialization</strong>&lt;br&gt;Gwen Binder (invited), Adaptimmune</td>
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<td>17:15 – 17:35</td>
<td><strong>Development of a bioreactor process for the production of NK-92 cells for allogeneic immunotherapies</strong>&lt;br&gt;Ricardo Baptista, Centre for Commercialization of Regenerative Medicine (CCRM), Canada</td>
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<td>17:35</td>
<td><strong>Free Evening /Dinner on your own</strong></td>
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Wednesday, January 8, 2014

07:30 – 08:30  Breakfast

**Session 6: Scale-up of Cell Therapy Processes**
Chairs: Michael Kallos (University of Calgary)
        Chris Hewitt (Loughborough University)
        Tom Brieva (Celgene Cellular Therapeutics)

08:30 – 08:55  Physical characterisation of the microbioreactor 'ambr' and Implications for animal and stem cell culture
Alvin Nienow (invited), Loughborough University, University of Birmingham, United Kingdom

08:55 – 09:20  Evolution of a scale-down model for generation of HIV-1 based Lentiviral vectors for use in ex-vivo manufacturing of cell therapy products
Robert Kutner (invited), Bluebird Bio, USA

09:20 – 09:40  Expansion and harvest of human mesenchymal stem cells from microcarriers in a stirred-tank bioreactor
Qasim A. Rafiq, Loughborough University, United Kingdom

09:40 – 10:00  Scalable suspension culture technologies to enable robust stem cell biomanufacturing
Todd C. McDevitt, Georgia Institute of Technology, USA

10:00 – 10:20  The development of a clinical manufacturing process for the *ex vivo* expansion of umbilical cord blood derived haematopoietic stem cells
Elizabeth Csaszar, Centre for Commercialization of Regenerative Medicine (CCRM), Canada

10:20 – 11:00  Coffee break
*Sponsored by Celgene*

**Session 7: Analytics and Product Characterization**
Chairs: Jeffrey Karp (MIT)
        Mark Lowdell (Royal Free Hospital)

11:00 – 11:25  Innovating preclinical drug discovery and human cell therapy
Steven Minger (invited), GE Healthcare, USA

11:25 – 11:50  Automating process control by using innovative label-free quantitative imaging
Philip Mathuis (invited), Ovizio Imaging Systems, Germany

11:50 – 12:10  Downstream processing of therapeutic cells - high-throughput process development for cell separation in aqueous two-phase systems
Sarah Nagel, Karlsruhe Institute of Technology, Germany

12:10 – 12:30  A metabolic approach to optimizing human mesenchymal stem cell expansion in cell therapy
Teng Ma, Florida State University, USA

12:30 – 12:50  Lysine deacetylase (KDAC) enzyme activity in cell differentiation
Teresa A. DeLuca, Northwestern University, USA
Human mesenchymal stem cells: Characterization and potency
Alexander K. C. Chan, Loughborough University, UK

Lunch

Free time / Networking

Session 8: Tissue Engineering, New Technologies and Tools
Chairs: Todd McDevitt (Georgia Institute of Technology) Devyn Smith (Neucentis)

Molecular engineering of synthetic microenvironments for stem cell culture
David Schaffer (invited), University of California at Berkeley, USA

Cell therapy scale-out: Parallel iPSC manufacture and differentiation for the delivery of immunomatched cell therapies
Emile Nuwaysir (invited), Cellular Dynamics International

Dynamic transcription factor activity profiles and inferred networks reveal key regulatory interactions during megakaryocytic and erythroid differentiation of bipotent progenitor cells
William M. Miller, Northwestern University, USA

Using novel non-invasive imaging as a process analytical tool for cell based therapy manufacturing
David Smith, Loughborough University, United Kingdom

Process development strategies to enable large scale fabrication of scaffold-less 3D ligament constructs for ACL reconstruction
Michael J. Smietana, University of Michigan, USA

Presentation of ECI Award for Scale-up and Manufacturing of Cell Based Therapies to Bob Nerem, Georgia Institute of Technology

Award Lecture
The challenge ahead: Cell-based therapies and the translation of bench-top research into products and clinical therapies
Bob Nerem, Georgia Institute of Technology, USA

Conference Banquet

Social Hour / Poster Session
Thursday, January 9, 2014

08:00 – 09:30 Breakfast and departures
1. **The development of scale-up bioreactor system for human induced pluripotent stem cell stirred suspension culture**  
   Masanori Wada, ABLE Corporation, Japan

2. **mRNA transfection in cell therapy: A step in vitro, a leap in vivo**  
   Kelvin S. Ng, Brigham & Women's Hospital, Harvard Medical School, USA

3. **A scalable modeling approach for the design and operation of a continuous fluidized-bed centrifuge for cell concentration and washing**  
   John C. Gaut, Celgene Cellular Therapeutics, USA

4. **Development of a harvest process for stirred tank microcarrier culture of therapeutic placental-derived cells**  
   David Hsiung, Celgene Cellular Therapeutics, USA

5. **Cell culture medium characterization and optimization by Design of Experiments (DOE) for the production of a placental-derived cellular therapy**  
   Andrea L. Nordberg, Celgene Cellular Therapeutics, USA

6. **Large scale ex vivo generation of red blood cells from human umbilical cord blood-derived hematopoietic stem cells**  
   Rajarajeswari Sivalenka, Celgene Cellular Therapeutics, USA

7. **Cell selection in cellular therapy and other large-scale industrial cell purification settings**  
   Ruud Hulspas, Cytonome/ST, LLC, USA

8. **T lymphocytes expanded in the WAVE bioreactor maintain a healthy phenotype**  
   Michelle Janas, GE Healthcare UK Limited, United Kingdom

9. **Development of a subculture equipment for a mass cell production in automated 3-dimensional tissue fabrication system (Tissue Factory)**  
   Toyoshige Kobayashi, Hitachi, Japan

10. **Novel human dopaminergic 3D in vitro model for pre-clinical assessment of gene therapy strategies**  
    Paula M. Alves, IBET and ITQB-UNL, Portugal

11. **Highly functional hepatic spheroids: Synergistic roles of microencapsulation and 3D configuration for differentiation of hepatic cells**  
    Paula M. Alves, IBET/ITQB-UNL, Portugal

12. **Evaluation of microcarrier-based suspension cultures for human mesenchymal stem/stromal cells**  
    Cláudia Lobato da Silva, Instituto Superior Técnico, Universidade de Lisboa, Portugal

13. **Serum-free media development for ex vivo expansion, differentiation, and cryopreservation of human mesenchymal stem/stromal cells**  
    Annie Ngo, Irvine Scientific, USA
14. Establishment of biological activity assays to qualify and reliably measure key growth factors derived from animal component free processes
   Ryan G. Linfield, Irvine Scientific, USA

15. Process development and scale-up of an allogenic cell therapy product
   Kostadinka (Koki) Lilova, Janssen R&D, USA

16. Low temperature cell pausing: An alternative cell preservation method for use in cell therapies
   Thomas Heathman, Loughborough University, United Kingdom

17. Systematic development of a process control system for the manufacture of human mesenchymal stem cells on microcarriers
   Thomas Heathman, Loughborough University, United Kingdom

18. Informing value driven cell therapy new product development
   Mark J. McCall, Loughborough University, United Kingdom

19. Immunoaffinity aqueous two-phase systems with pegylated CD133 antibodies for the potential recovery of stem cells
   Marco Rito-Palomares, Tec de Monterrey, Mexico

20. The quality stability for human epithelial cell sheet after transportation by air
   Toshiyuki Owaki, Tokyo Women's Medical University, Japan

21. Allogeneic cell therapy bioprocess economics and optimization: Single-use volume reduction technologies
   Sally Hassan, University College London, United Kingdom

22. Induced pluripotent stem cell processing for drug discovery platforms: Process economics and optimisation
   Michael Jenkins, University College London, United Kingdom

23. Using a Design of Experiment (DoE) approach to optimise pluripotent stem cell differentiation for subsequent manufacturing
   Iwan T. Roberts, University College London, United Kingdom

24. IPS derived photoreceptor production in an agitated suspension culture system
   Vishal Sharma, University College London, United Kingdom

25. Comparison of filtration and centrifugation for cryoprotectant removal from thawed cell suspensions
   Rui Tostoes, University College London, United Kingdom

26. Purification challenges for whole cell therapies: The isolation of photoreceptor precursors for treatment of retinal dystrophy
   Ben Weil, University College London, United Kingdom

27. Rice Bran Extract (RBE) as supplement for Mesenchymal Stem Cells (MSCs)
   Satoshi Terada, University of Fukui, Japan

28. A parallel bioreactor system for investigating metabolic pathway changes during iPSC reprogramming
   Yonatan Lipsitz, University of Toronto, Canada
29. Towards scalable production and cryopreservation of functional iPSC-derived cardiomyocytes
   Cláudia Correia, IBET/ITQB-UNL, Portugal

30. Towards a robust and scalable bioprocess for the expansion of human pluripotent stem cells
   Cláudia Correia, IBET and ITQB-UNL, Portugal

31. The development of laminar flow-based suspension culture system for human iPS cells and the application to the cardiac tissue engineering
   Katsuhisa Matsuura, Tokyo Women’s Medical University, Japan

32. Ultra scale-down approach to membrane separation procedure of human cells for therapy: Effect of cell concentration on cell loss
   Fernanda Masri, University College London, United Kingdom

33. Concentration and harvest of hepatic progenitor stem cells using the integrity
   Fabien Moncaubeig, ATMI LifeSciences, Belgium

34. Generating aligned vascular networks via a scalable process
   Jacob Ceccarelli, University of Michigan, USA

35. Decoding human cardiac stem cell proteomic profiles towards the design of efficient therapies for cardiac repair
   Margarida Serra, IBET/ITQB-UNL, Portugal

36. Designing scalable and clinical-grade filtration-based strategies for the downstream processing of human mesenchymal stem cells
   Margarida Serra, ITQB-UNL/IBET, Portugal

37. Statistical analysis of process consistency for allogeneic cardiosphere-derived cells
   Brandon J. Burton, Capricor Inc., USA