ISCT ANZ Regional Meeting
in association with the
Cooperative Research Centre for
Cell Therapy Manufacturing

Program

TRANSLATING CELL THERAPY 2015

October 16-17, 2015
Adelaide, South Australia
TRANSLATING CELL THERAPY 2015

16-17 October, 2015
ADELAIDE

INTERNATIONAL SOCIETY FOR CELLULAR THERAPY:
ISCT is a global association driving the translation of scientific research to deliver innovative cellular therapies to patients. ISCT is the only group focused on pre-clinical and translational aspects of developing cell therapy products. As such, ISCT helps academic, government and biotech/pharma sectors transform research into practice and product.

AUSTRALIA AND NEW ZEALAND REGIONAL MEETINGS:
Past regional meetings were held in Adelaide, SA (2009), Margaret River, WA (2010), Sydney, NSW with HAA (2011), Sydney with ATBF (2012) and Lorne with ASSCR (2014). In 2013 we were fortunate to have the ISCT annual scientific meeting in the region in Auckland. The upcoming Regional Meeting in Adelaide again promises to deliver an exciting opportunity to leverage your company’s exposure to leading cell therapy professionals in the region.

THE COOPERATIVE RESEARCH CENTRE FOR CELL THERAPY MANUFACTURING:
The Cooperative Research Centre for Cell Therapy Manufacturing (CTM CRC) is a collaboration of 15 research and industry participants. Through this collaboration CTM CRC will develop cost-effective manufacturing and rapid translation of cell therapies into clinical practice for the treatment of conditions such as diabetes, chronic wounds, cardiovascular disease, and immunotherapies.

CTM CRC also has a dedicated cGMP manufacturing facility designed to deliver cell-based therapeutics, which provides the infrastructure necessary for the CRC’s first-in-man clinical trials.
ISCT ANZ Regional Meeting
16-17 October, 2015
Adelaide, South Australia

The International Society for Cellular Therapy ANZ regional committee together with the Cooperative Research Centre for Cell Therapy Manufacturing welcome you to our joint meeting in Adelaide. The program will focus on sharing lessons learned from cell-based therapies in early phase development. The meeting covers clinical manufacturing, process improvement and technologies, quality control, quality systems, and reimbursement strategies. Included is a special session showcasing our submitted papers, and the whole meeting is designed to maximise interaction and discussion between delegates. Before and after the meeting visit our new website http://isctanz.org.au/ for information on local initiatives. Welcome to Adelaide!

Janet Macpherson, Chair, Translating Cell Therapy 2015
NextCell
CGMP Cell & Tissue Therapies

- Research refinement
- Process optimisation
- Technology Transfer

QUALITY ASSURANCE DEVELOPMENT
QUALITY CONTROL

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CGMP cell manufacturing
Early stage clinical trials

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NextCell Pty Ltd
Level 3, Building MM, UniSA Mawson Lakes, South Australia
**PROGRAM**

**Friday 16 OCTOBER**

Diamond Room, Mayfair Hotel, 45 King William Street, Adelaide SA 5000

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<thead>
<tr>
<th>TIME</th>
<th>Friday 16 OCTOBER</th>
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<tbody>
<tr>
<td>18:00 – 19:30</td>
<td>Registration Desk Open at Mayfair Hotel</td>
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<tr>
<td>18:15 – 18:30</td>
<td><strong>Translating Cell Therapy 2015</strong></td>
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<tr>
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<td>Meeting Opening</td>
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<td>Official Welcome – Sherry Kothari &amp; Robert Nordon</td>
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<td>Special Guest Speaker - Professor John Rasko</td>
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<td>18:30 – 21:00</td>
<td><strong>Translating Cell Therapy 2015</strong></td>
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<td>Welcome Reception</td>
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<td>Canapes and beverages</td>
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**Saturday 17 OCTOBER**

Bradley Forum, Hawke Building City West Campus, Uni SA, SA

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<thead>
<tr>
<th>TIME</th>
<th>Saturday 17 OCTOBER</th>
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<tbody>
<tr>
<td>7:30 – 9:30</td>
<td>Registration Desk Open</td>
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<tr>
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<td>Exhibitor set-up</td>
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<td>Please note – there is no breakfast on site</td>
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<tr>
<td>08:00 – 08:30</td>
<td><strong>Welcome and meeting overview</strong></td>
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<tr>
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<td>Thank you to Sponsors of Translating Cell Therapy 2015</td>
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<tr>
<td></td>
<td>Naomi Morrison, Thermo Fisher Scientific</td>
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<tr>
<td>08:30-10:30</td>
<td><strong>Session 1: Manufacturing Cells for the Clinic</strong></td>
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<td>Chairs: Lynn O’Donnell and Vicki Antonenas</td>
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<td>08:30</td>
<td><strong>Dr Shelly Heimfeld</strong>, Fred Hutchinson Cancer Research Centre, Seattle, USA</td>
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<td><em>Therapeutic Cell Production in an Academic Setting</em></td>
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<td>09:00</td>
<td><strong>Marian Sturm</strong>, Cell &amp; Tissue Therapies Western Australia, WA</td>
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<td><em>Allogeneic MSC for proof of concept immune modulation studies</em></td>
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<td>09:20</td>
<td><strong>Leighton Clancy</strong>, Sydney Cellular Therapies Laboratory, Westmead Hospital, NSW</td>
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<td><em>Third-party partially HLA-matched virus-specific T cells to treat refractory viral infections</em></td>
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<td>09:40</td>
<td><strong>Siok Tey</strong>, QIMR Berghofer, QLD</td>
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<td><em>A phase I study of Haploidentical haematopoietic stem cell transplantation with add-back of iCasp9 safety switch gene-modified donor T cells</em></td>
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Translating Cell Therapy 2015
<table>
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<tr>
<th>Time</th>
<th>Session/Activity</th>
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<tr>
<td>10:00 – 10:30</td>
<td>Morning tea with trade display</td>
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| 10:30 – 12:30 | Session 2: Practical Considerations for Cell Therapy Manufacturing  
Chair: Pam Dyson and Marian Sturm  
Allan Chen - Bioprocessing Technology Institute, A*STAR, Singapore  
*Bioprocessing aspects of expansion and differentiation of human pluripotent stem cells*  
Rob Short, Advanced Therapeutic Technologies, UniSA, SA  
*Smart surfaces and materials advancing cost effective cell therapy*  
Simon Barry, Women’s and Children’s Hospital, Adelaide, SA  
*Novel T cell expansion technology and biomarkers for human T cell therapy*  
Lynn O’Donnell, James Cancer Center, Ohio State University, USA  
*Cell Processing in the 21st Century*  
12:00 – 13:00 | Lunch with trade display and Concurrent Poster Session |
| 13:00 – 14:00 | Session 3: Career Development Workshop  
Chairs: Shelly Heimfeld and John Rasko  
Selected abstracts x 5 minutes (3+2) talk to poster  
Susan Best, NPAAC requirement for HIV, HCV and HBV nucleic acid testing in processing of HPC.  
Uwe Hattenhorst, Towards a Fully Automated Process to Reduce Alloreactivity of Allogeneic Apheresis Products by the Depletion of CD45RA-Positive Cells.  
James Favaloro, Magnetic separation does not provide adequate purity for use in molecular chimerism analysis.  
Matthew Delgado, Expansion of ISBT 128 Terminology to Regenerated and Source Products  
Zlatibor Velickovic, Validation and implementation of a cost effective electronic document control system  
Caroline Gargett, A83-01, a Small Molecule TGF-beta Receptor Inhibitor Maintains Mesenchymal Stem Cell Phenotype and Function during Serum-Free Culture Expansion  
Louise Smith, Developing a dressing for topical delivery of Multistem® cells to wounds  
Tessa Gargett, Chimeric Antigen Receptor T Cells as an Immunotherapy for Advanced Melanoma  
Kajal Chaudhry, Live imaging of anti-tumour responses induced by CAR19 T-cells |
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<tr>
<th>Time</th>
<th>Session/Panel</th>
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<tr>
<td>14:00 - 15:15</td>
<td>Session 4: Making Successful Cell Therapy Products (Pay)</td>
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<td>Chairs: Janet Macpherson and William Mercer</td>
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<td>14:00 - 14:20</td>
<td>Jeanette Ripper Cell Therapies Pty Ltd, Melbourne, VIC</td>
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<td>Designing a quality strategy</td>
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<td>14:20 - 14:40</td>
<td>Dawn Driscoll DCi Botech Inc, USA</td>
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<td>Designing a reimbursement strategy</td>
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<td>14:40 - 15:00</td>
<td>Sue O’Malley Medical Intelligence, NSW</td>
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<td>Implementing reimbursement</td>
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<td>15:00 - 15:30</td>
<td>Afternoon tea with trade display</td>
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<td>15:30 - 17:00</td>
<td>Session 5: Strategies for Australian Market Development</td>
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<td>Chairs: Robert Nordon and Dominic Wall</td>
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<td>15:30 - 16:00</td>
<td>Michael May, CCRM Canada</td>
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<td>Cell Therapy in Canada today</td>
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<td>16:00 - 16:30</td>
<td>William Mercer</td>
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<td>Partnering with medical device companies to address unmet needs in cell</td>
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<td>manufacturing</td>
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<td>16:30 - 17:00</td>
<td>Sherry Kothari, Cell Therapy Manufacturing CRC, Adelaide</td>
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<td></td>
<td>Cell Therapy in Australia today</td>
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<td>17:00 - 17:45</td>
<td>Panel Discussion:</td>
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<td>17:45 - 18:00</td>
<td>Session 6: Awards Presentation and Closing Address</td>
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<td></td>
<td>Janet Macpherson and TBC (CRC representative)</td>
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<td>18:00 - 19:30</td>
<td>Closing Reception &amp; Networking Opportunity</td>
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ICCBA: Setting Standards for the Safe Transfer of Cellular Therapy Products Worldwide

According to the WMDA, nearly 50% of blood stem cell donations cross an international boundary for patients in need. With registries in over 50 countries representing more than 50 official languages, the need for standardization of terminology has never been higher. Recognizing this need, the accreditation organizations AABB, FACT, and JACIE all require ISBT 128 implementation plans in their current standards, and aim to incorporate full implementation in future editions.

The ISBT 128 Standard, development and managed by ICCBA, is the sole global standard for the identification and coding of medical products of human origin. Recognized by the World Health Organization, ISBT 128 for cellular therapy is currently being used by 458 organizations in 56 countries. There are now over 1,600 standardized cellular therapy Product Description Codes, with new requests processed monthly.

To learn more about ISBT 128, contact our office today!

Contact us for more information:
iccbbaha@iccbba.org
+1 909-793-6516

ICCBA
P.O. Box 11309
San Bernardino, CA 92423-1309, USA

Translating Cell Therapy 2015
Therapeutic Cell Production in an Academic Setting
Shelly Heimfeld, Fred Hutchinson Cancer Research Center, USA

The Fred Hutchinson Cancer Research Center (FHCRC) offers a broad range of facilities and qualified trained personnel to support the development and manufacturing of novel and innovative cell-based therapies for Phase I/II clinical testing. GMP capabilities include specialized equipment and uniquely trained staffing necessary to support clinical studies that incorporate specific cell selection, genetic modification and/or ex vivo manipulation of patient stem cells, dendritic cells and T-cells, as well as the production of research and clinical grade monoclonal antibodies, fusion proteins, vaccines, lentiviral vectors and other therapeutic molecules. These facilities assist the Center’s investigators in pre-clinical research, pilot production, and clinical trial design, and provide the support necessary for filing of both Investigational New Drug (IND) and Investigational Device Exemption (IDE) Applications and Drug Master Files (DMF). Once approval from the regulatory agencies is obtained, clinical manufacturing commences, with appropriate levels of quality control, quality assurance, and regulatory oversight. Currently the FHCRC has over 20 active IND and IDE studies, ranging from cell selection protocols to more extensive manipulations involving genetic modifications, ex-vivo expansion, and antigen-specific immunotherapies. Examples of specific studies will be presented, along with some of the unique challenges that arise when operating such a complex operation within the constraints of an academic institution.
Allogeneic MSC for Proof of Concept Immune Modulation Studies

Marian Sturm, Royal Perth Hospital, WA

It has been a decade since the first reported clinical use of mesenchymal stromal cells (MSC) for the treatment of GVHD but translation of this therapy has been painstakingly slow. In part, scepticism developed as a result of disappointing Phase III data from the commercial sector. However, many hospital based groups have continued to progress MSC therapy with the belief that MSC have great potential to mollify immune disorders by modulating immune and inflammatory responses and inducing immune tolerance. Proof of concept of allogeneic MSC therapy lays in the demonstration of clinical efficacy through clinical trials.

Our centre began manufacturing allogeneic bone marrow derived MSC in 2007 and now manufactures under TGA licence and FACT accreditation. To date, over 100 patients have received MSC therapy for immune related disorders. The majority of patients have been enrolled in one of 6 clinical trials but 23 patients have also accessed MSC therapy on compassionate grounds. The trials range from initial Phase I studies to Phase 2a and 2b trials and cover the array of immune disorders of graft versus host disease (GVHD, allogeneic bone marrow transplant related), host versus graft disease (organ transplant rejection) and autoimmune disease. The trials include a Phase I steroid refractory GVHD, Phase 2b randomised steroid naïve GVHD, Phase I refractory acute kidney rejection and Phase 2a chronic lung rejection, and a Phase 2 in Crohn’s disease. Patients with other autoimmune disorders have been treated on compassionate grounds and pave the way for additional clinical trials.

MSC are delivered intravenously according to the dosing schedule of the trial, usually 2 x 106 MSC/kg patient weight for 2 or 4 infusions, with additional doses if clinically indicated. Over 600 infusions of CTTWA manufactured MSC have been performed with no serious related adverse events observed. The first patient treated with CTTWA MSC therapy is greater than 6 years out and in remission for GVHD.

Collectively, the evidence from our various studies demonstrates that the MSC therapy is safe and is a therapeutic option for immune modulation. Further late phase trials are in planning to complete proof of concept.

Translating Cell Therapy 2015
Third-party partially HLA-matched virus-specific T cells to treat refractory viral infections

Leighton Clancy, Emily Blyth, Babara Winters, Jane Burgess, Renee Simms, Chun Kei Kris Ma, Kenneth Micklethwaite and David Gottlieb, Sydney Cellular Therapies Laboratory, Westmead Hospital, Sydney, NSW

Introduction: Adoptive transfer of donor derived virus specific T cells can be effective therapy for infections in allogeneic stem cell transplant recipients. However, this is not a practical strategy to treat acute infections due to the time required to prepare products and potential donor unavailability. To overcome this, treatment with cryopreserved partially HLA matched T cells from third party donors are being investigated. Recent reports describe disease resolution using cells matched at only one or two HLA alleles. This less stringent requirement for matching would allow a small bank of cells to provide most patients with a potential product. We describe the establishment of a T cell bank to treat patients who have failed antiviral pharmacotherapy.

Methods: Products were generated by co-culturing PBMC with dendritic cells loaded with overlapping peptides derived from CMV pp65, adenovirus hexon or EBV BZLF1, LMP2A and EBNA1 proteins. Cultures were re-stimulated once and cultured for 14 days with IL-2 prior to cryopreservation and specificity testing. Patients with persistent viremia or tissue infection after 2 weeks standard therapy were eligible to receive partially HLA matched third-party derived virus specific T cells and were followed for 12 months.

Results and Discussion: T cell products were expanded from 31 donors to create a bank of 218 bags of virus specific T cells (110 targeting CMV, 53 AdV and 55 EBV). CMV specificity could be mapped to several immunodominant pp65 epitopes restricted to common HLA types including HLA-A*0101, HLA-A*0201, HLA-A*2402, HLA-B*0702, HLA-B*3501. AdV T cells primarily elicited CD4 mediated responses however specificity could be mapped to CD8 epitopes restricted to HLA-A*0101 and HLA-A*2402 as well as 14 different CD4 T cell epitopes restricted to common HLA-DRB1 alleles. Specificity of EBV products to the three antigens was highly variable and donor dependent. Based on HLA frequency analysis we estimate 94%, 89% and 74% of patients would have access to a CMV, AdV and EBV specific product respectively with the current bank. Nineteen patients (17 CMV, 1 EBV, 1 ADV) from 6 transplant centres have received 31 infusions in total. There were no immediate infusion toxicities. No patients developed acute GVHD. 16 patients could be assessed for viral response with 12 complete responses (virus PCR negative) and 4 partial responses (>50% viral titre reduction). Banked partially HLA matched virus specific T cells appear safe and may contribute to viral control though further investigations are required to determine the persistence of infused cells and mechanism of viral control.
A phase I study of haploidentical haematopoietic stem cell transplantation with add-back of iCasp9 safety switch gene-modified donor T cells

Siok Tey, QIMR Berghofer Medical Research Institute, QLD

Inducible caspase 9 (iCasp9) is a safety switch that enables the conditional elimination of transferred cells in the event of unwanted effects. Its first-in-human proof-of-principle came in the field of paediatric T cell-deplete haploidentical haematopoietic stem cell transplantation. Here, iCasp9-transduced donor T cell were added back to accelerate immune reconstitution and patients who developed graft-versus-host disease (GVHD) were given a small-molecule dimerizer, AP1903, which rapidly triggered apoptosis of iCasp9-transduced T cells and abrogated GVHD.

We opened a phase I study to determine the safety of iCasp9 T cell addback in adults (age 18-59) undergoing myeloablative haploidentical transplantation. Myeloablative conditioning consists of 8Gy TBI on d-9, thiopeta (5 mg/kg/day) on d-8 & -7; fludarabine (40 mg/m2/day) on d-7 to -3; and rabbit ATG (1.5mg/kg/day) on d-5 to -2. Donor GCSF-mobilised PBSC are CD34+-selected and infused fresh on day 0 without post transplant immunosuppression or G-CSF. On day ≥21, a dose of iCasp9-transduced donor T cells is infused, with dose escalation in cohort size of two: 5x10e5/kg, 1x10e6/kg, 5x10e6/kg and 1x10e7/kg. Patients who develop GVHD grade ≥II are treated with AP1903.

Three patients have been enrolled to date. The first two patients, both with high risk acute myeloid leukaemia, have received iCasp9 T cells at 5x10e5/kg on day 24 - 25. The transgenic T cells were readily detected by flow cytometry and have persisted for >160 days post infusion. Pt#1 received a second infusion (1x10e6/kg) for mixed T cell chimerism. Pt#2 received two further infusions (1x10e6/kg and 4x10e6/kg) for disease relapse. Neither patients have had GVHD or other adverse events as a result of the T cells. Both patients had CMV reactivation and self-limiting BK viruria before T cell addback but neither have had invasive infections to date. Recruitment to this study is ongoing.

Gene-modified T cells that can be tracked in vivo and conditionally eliminated has a number of potential research and clinical applications in allogeneic transplantation and we look to investigate some of these now that we have the capacity to locally manufacture iCasp9-transduced donor T cells suitable for phase I studies.
Bioprocessing aspects of expansion and differentiation of human pluripotent stem cells

Allen Chen, Bioprocessing Technology Institute :: Agency for Science, Technology and Research (A*STAR) Singapore

Human pluripotent stem cells (hPSCs) which includes human embryonic and induced pluripotent stem cells, are unique in their ability of self-renewing and differentiation into a wide range of cell types. These unique properties enabled the use of hPSC in drug toxicity testing and discovery, disease modeling, tissue engineering and cell therapy in regenerative medicine. However, one of the major hurdles that limits their widespread application is the need for development of an efficient scalable bioprocess platform for large scale hPSC expansion and differentiation, that needs to comply with current Good and Manufacturing Practice conditions. To date, the expansion of hPSCs is mainly carried out in traditional tissue culture plates and can only be used when low doses of cells are required. The demand for large quantity of cells has reinvigorated interest in establishing suspension culture in stirred bioreactors similar to the ones currently used by industry for the production of biologics. Two methods for establishing suspension agitated based hPSC culture have been reported: microcarriers and cell-aggregates. This presentation will summarize and discuss the latest findings of the planar, microcarrier and cell-aggregate based platforms with emphasis on microcarrier based cultures.

Microcarriers have been developed for culturing adherent cell lines mainly for vaccine production and research purposes. Typical commercial microcarriers are spherical with smooth surfaces that allows the growth of adherent mesenchymal stem cells as monolayers similar to the planar cultures. However, hPSCs which grow in planar cultures as multi-layer colonies generates cell/microcarrier aggregates in microcarrier cultures. Critical parameters that affect hPSC expansion like microcarrier type, coating of the microcarriers with extracellular matrices, cell seeding conditions, medium development and improved bioprocess parameters will be discussed in this presentation.

Differentiation of expanded hPSC in planar or embryoid body cultures are limited in scale-up due to either limitation of surface area available for the cells or the difficulties in embryoid body formation and maintenance. On the other hand, since hPSC grows as controlled size cell/microcarrier aggregates in stirred bioreactors, they can be directly differentiated in the same bioreactor simply by changing media and using specific differentiation inducers. hPSC differentiation on microcarriers is still considered a new approach with only few examples demonstrating the generation of cardiomyocytes and neural progenitors in stirred microcarrier cultures. The challenges of achieving such integrated, scalable processes where hPSCs expansion and differentiation are combined seamlessly into one unit operation will be also discussed.
Smart surfaces and materials advancing cost effective cell therapy

Rob Short, UniSA, SA

This presentation will highlight how smart surfaces and materials processing could potentially impact on the COGs and the effective delivery of cells to patients. Some examples will be drawn from the work ongoing at the Cell Therapy Manufacturing CRC, whereby challenges in cell expansion and delivery are being met by the processing of FDA approved material to significantly enhance their performance. For example, the localization of cells at the site of injury has the potential to reduce cell number and produce a direct paracrine effect. This requires bespoke delivery devices. Preclinical data are now demonstrating the potential of this approach.
Novel T cell expansion technology and biomarkers for human T cell therapy

Molecular Immunology, WCHRI, Gastroenterology, WCHN, Robinson Research Institute, CRC-CTM, University of Adelaide, SA

The demand for affordable cell therapy is rapidly rising, and the clinical imperative for tailored therapies with few side effects has placed cell therapy at the vanguard of modern medicine. While there is early promise for the use of T cells in cell therapy, the cost of manufacturing remains a hurdle to the wide scale use of cell therapy. The unmet need includes tolerisation in solid organ transplantation, prevention of graft vs. host disease in bone marrow transplantation autoimmune disease and cancer immunotherapy, which together account for thousands of patients a year who could benefit from cell therapy. In order to address this we are developing functionalised smart surface technologies that can be used in large scale expansion of T cells for clinical use. We have combined melt electrospray 3D scaffold production, epoxy plasma polymerisation and biological stimulation of T cells using surface immobilised antibodies to develop a T cell expansion platform that is scalable and affordable. Pilot data on this smart surface technology will be presented showing its ability to expand CD4+ T cells, regulatory T cells and CD8 T cells, giving utility for several immunotherapy approaches.

As part of a program to develop better biomarkers for immunotherapy cell isolation, we have also identified a novel biomarker for human T cells. Natural Treg express the transcription factor FOXP3, but isolation using FOXP3 is not tractable for functional assays or for cell enrichment. A cell surface surrogate for FOXP3 is hence required, and such biomarkers are critical for understanding immune homeostasis and its breakdown in disease. To discover novel human Treg cell surface molecules we performed a genome wide analysis of differential gene expression (Sadlon et al JI, 185(2):1071-81). In search of a biomarker surrogate for FOXP3, we mined for up-regulated novel surface proteins, and Peptidase Inhibitor 16 (PI16) was identified. Analysis of resting and stimulated Treg and Thelper cells demonstrated that PI16 was readily detectable on the surface of resting nTreg. In conjunction with CD25+, PI16 is able to identify 20-80% of both resting and stimulated FOXP3+ cells, and a similar proportion of resting CD25- cells. Detailed characterisation of PI16+ve Treg cells reveals an antigen experienced memory phenotype. PI16+ CD25+ cells are highly suppressive in vitro and preliminary data suggest that they may be more potent than the CD25+ Treg pool. Importantly, stimulation of CD25-Thelper cells, which substantially up regulates CD25 expression and transiently induces FOXP3, does not induce detectable expression of PI16, suggesting that PI16 is able to segregate Treg subsets, as it is not expressed on CD25- derived iTreg. When a cohort of 30 type 1 diabetes samples were analysed for PI16+ Treg FOXP3 expression, we observed a significant decrease in FOXP3 expression levels in t1d, suggesting a loss of functional fitness in these Treg. We propose that PI16 identifies functional FOXP3+ve Treg and FOXP3-ve Thelper subsets with clinically relevant functional capacity in humans.
Cell Processing in the 21st Century: Creative Approaches to Timely and Timeless Operational Challenges

Lynn O'Donnell, Ohio State University, USA

The cell therapy industry is poised to expand at an unprecedented pace over the next decade. If you work in a cell therapy processing facility, you may be experiencing anything from blissful ignorance to panic attacks and insomnia, depending on how closely you follow industry trends and what is happening right now in your own facility. You probably also have your own set of current issues that prevent you from thinking much about the future. Overcoming both long-running and new challenges will require the ability to think outside the box. This session will provide an overview of the operational needs, challenges and possible solutions to deal with a rapidly growing and changing industry, such as staffing and productivity models, succession planning, working with new clinical customers, preparing budgets for process development and clinical trials, working with executive management and fundraising. More idealogical concepts will also be presented, including how to encourage creative thinking and how to balance both perspective and prudence in the face of increasing oversight. Audience participation will be welcome, as collective ingenuity can always lead to better solutions.
Designing a Quality Strategy for Cellular Therapy Products
Jeanette Ripper, Cell Therapies Pty Ltd, VIC

The commercialization of a cell therapy product requires consideration of several unique issues, including those posed by the cell therapy itself as a complex living, dynamic product. The safety and efficacy of the product must first be established in clinical trials, with subsequent commercial manufacturing requiring submission and approval of a product dossier, and application and granting of a Product Manufacturing Licence, to demonstrate compliance with Good Manufacturing Practices (GMPs).

It is the regulatory requirements that determine much of the commercial manufacturing process, particularly how it is controlled and documented, and the infrastructure needed to support it. In order to streamline the regulatory pathway for new products, consideration should be given to introducing principles of good manufacturing design early into the cell therapy manufacturing process, including defining and documenting the critical product attributes and taking an integrated approach towards the early development of quality parameters such as process validation and in-process controls. This presentation will discuss the establishment of a quality strategy for a cell therapy product, to ensure that product consistency is carried through from R&D to commercialisation and that the manufacturing process supports the production of safe and effective cellular therapeutics.
Designing Cell Therapy Trials for Regulatory Approval and Reimbursement
Dawn Driscoll, DCi Biotech Inc, USA

Abstract: We generally focus our clinical trial design on generating answers to the questions Regulators, health care providers and patients will ask. We also need to design trials that will generate data to address the questions that payers, whether public or private, will need to have answered before granting reimbursement for the therapy. Ideally, multiple regions’ requirements can be met within one well designed phase 3 study. This talk will present basic concepts in late stage trial design for international commercialization, focused on reimbursement.
Reimbursement in Australia: How hard can it be?

Sue O'Malley, Medical Intelligence, NSW

In Australia, all medical services need to be covered by the Medicare Benefits Schedule (MBS). The importance of having the 'right' evidence for a successful reimbursement submission in Australia for new medical services is evident by the high percentage of unsuccessful applications for MBS funding over recent years. A review of recent submissions is used to illustrate the main reasons for failure to get MBS funding and what is required for a successful submission. An outline of the MBS funding application process, including timelines and data requirements is also presented. Finally, a look at cell therapy and any 'special' evidence requirement.

Translating Cell Therapy 2015
Cell Therapy in Canada today
Michael May, CCRM, Canada
Abstract not available

Partnering with medical device companies to address unmet needs in cell manufacturing
Bill Mercer, USA
In recent years scientific innovation has delivered some exciting clinical results in fields of cell and regenerative medicine. Financial resources both public, private and academic are flowing into this segment like never before. Moreover, organizations are looking for returns on that invested capital. Traditional manufacturing technologies and processes are for the most part manual requiring high cost clean rooms. Collectively, new systems and processes have to be developed that help ensure quality, scalability, sustainability and lower cost of goods. All of those being requirements in an era of heightened regulatory scrutiny.

The medical device industry is an important partner to big pharma, biotech and academia in their pursuit of an improved cell manufacturing system. The speaker will examine experiences from the past and suggest approaches for the future. Ultimately, this has to be a multi pronged approach to deliver these new life saving therapies to patients around the world. While an all-inclusive closed cell manufacturing system would be ideal, this will be many years away

Cell Therapy in Australia today
Sherry Kothari, Cell Therapy Manufacturing CRC, Adelaide, SA
Abstract not available
Registration and Abstract Submission

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ABSTRACTS – POSTERS

NPAAC requirement for HIV, HCV and HBV nucleic acid testing in processing of HPC

Susan Best. National Serology Reference Laboratory

The 2015 edition of the “Requirements for procedures related to the collection, processing, storage and issue of human haemopoietic progenitor cells” added a requirement for HIV, HCV and HBV nucleic acid testing (NAT) as a step in the processing of human progenitor cells. The publication indicated that this testing could be performed in a NATA accredited or TGA licensed laboratory. Laboratories holding either of these certifications would operate with appropriate levels of quality assurance to conduct this testing. However, because of the clients they serve, only TGA licensed laboratories are likely to routinely use in vitro diagnostic devices (IVDs) with appropriate registration on the Australian Register Therapeutic Goods (ARTG) for detection of HIV, HCV and HBV nucleic acid. This presentation will unravel the classification of infectious diseases IVDs on the ARTG and discuss the classification in the context of NAT for HPCs.

Towards a Fully Automated Process to Reduce Alloreactivity of Allogeneic Apheresis Products by the Depletion of CD45RA-Positive Cells

Stephanie Soltenborn, Joanna Strenzel, Julia Dzienek, Daniela Koch, Nadine Mocikel-Tenbrinck, Volker Huppert, Mario Assenmacher, Kirsten Langeveld and Uwe Eduard Hattenhorst

Milenyi Biotec GmbH, Bergisch Gladbach, Germany

Introduction: Memory T cells hold great potential for improving the outcome of allogeneic stem cell transplantation by providing defense against pathogens in immunocompromised individuals. In contrast, naive T cells contained in the transplant imply the highest risk of causing GVHD. Depletion of naive T cells from blood products may therefore avoid alloreactivity, while the presence of memory T cells might sustain the anti-infection potential of the transplant.

CD45RA is expressed on leukocyte subsets and distinct hematopoietic stem cells. It is present on the entire fraction of naive T cells but only on a small fraction of memory T cells. Therefore, CD45RA represents a good marker for the depletion of naive T cells.

Methods: The CE-certified CliniMACS CD45RA System enables depletion of naive T cells from leukapheresis products using a semi-automated process based on the CliniMACS Plus Instrument. Currently we are developing a fully automated process on the CliniMACS Prodigy, which allows cell processing in a closed GMP-compliant system. We tested two strategies involving a one-step or two-step procedure for CD45RA+ cell depletion.

Results: Using the CliniMACS Plus Instrument CD45RA+ cells were depleted from either non-mobilized or mobilized leukapheresis products. The leukapheresis products differed in WBC counts, whereas the frequencies of CD45RA+ cells among total WBC were comparable. After cell processing, recoveries and purities of CD45RO+ cells, and efficiencies of CD45RA+ cell depletion were similar for both apheresis products.

Non-mobilized leukapheresis products were split into two portions and each half was processed using one-step or two-step depletion procedures on the CliniMACS Prodigy. Comparable results were achieved for viability of CD45RO+ cells and WBC recovery. The two-step process resulted in a higher efficiency of CD45RA+ cell depletion.

Conclusion: Both the semi-automated process on the CliniMACS Plus Instrument and the fully automated CliniMACS Prodigy led to comparable results for depletion of naive T cells from leukapheresis products.

Magnetic separation does not provide adequate purity for use in molecular chimerism analysis.


Translating Cell Therapy 2015
Aim: To evaluate the suitability of magnetic separation as an alternative to FACS sorting for purifying leukocyte subsets for use in single cell molecular chimerism analysis.

Method: Fresh LiHep samples from healthy volunteers (n=6) and patients at various stages of haematopoietic recovery (n=12) were purified using an automated magnetic cell separation platform and evaluated for purity and recovery by flow cytometry.

Results: Purity of CD3 T cells isolated from fresh whole blood from patients (mean purity: 73.8%, range: 12.8-97.8, n=12) was significantly (t=2.23, p=0.04) lower than samples from healthy individuals (mean: 97.7%, range: 94.1-99.9, n=6).

Purity appeared to be related to starting CD3 numbers with significant positive correlation between CD3 purity and absolute number of CD3 cells present in loaded whole blood samples (n=16, r=0.63, p=0.006). Recovery of purified CD3 cells appeared independent of starting CD3 numbers, although significantly greater recovery was obtained when purifying samples from healthy donors as compared to patients who had undergone stem cell transplantation (t=4.94, p=0.0001). It should be noted that recovery showed a wide range for both normal donors (mean: 92.0%, range: 34.8-145.4, n=6) and BMT patients (mean: 21.6%, range: 1.9-55.3, n=6). The fact that half the samples from healthy donors showed greater than 100% recovery also warrants further investigation.

Conclusion: Single lineage chimerism is a useful tool, which can direct therapy. The current gold standard, molecular single cell chimerism analysis is dependent on cell purities in excess of 98%.

While magnetic separation performed reasonably with samples from healthy individuals, it failed to obtain adequate cell purity with leucopenic patient samples which represent significant proportion of clinical samples. The current method of cell sorting by flow cytometry is time consuming, however it provides unmatched purity required for this critical clinical application.

Status of ISBT 128 Implementation for Cellular Therapy Worldwide
Matthew Delgado, Paul Ashford and Pat Distler, ICCBBA

Aims/Background: In 2010, the World Health Assembly Resolution WHA63.22 urged member states “to encourage the implementation of globally consistent coding systems for human cells, tissues and organs as such in order to facilitate national and international traceability of materials of human origin for transplantation.” ISBT 128 is such a system, and, according to the WHO website, “is the sole global standard for the identification and coding of MPHO [Medical Products of Human Origin].”

Given the high frequency of international distribution of cellular therapy (CT) products, standardized coding and labeling is particularly important in CT to achieve efficient and accurate communication across language barriers.

The use of ISBT 128 terminology, coding, and labeling for CT products has been increasing steadily around the world. AABB, FACT, and JACIE require the use of ISBT 128 terminology and a plan for full implementation. Of interest in the coming year, ISBT 128 is compatible with the mandatory Single European Code.

Current Status: There has been a steady growth in CT facilities registered with ICCBBA to use ISBT 128.

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<th>Year</th>
<th>Number of CT facilities registered with ICCBBA</th>
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As of August 2015 651

110 ICCBBA-registered CT facilities in 25 countries responded to a recent survey on their status of implementation of ISBT 128. 52% indicated they have fully implemented ISBT 128 and another 28% indicated they are using ISBT terminology, but have not implemented ISBT 128 labeling. Of those who had not implemented, 66% indicated they planned to implement within a year, while 34% said they would implement within 2 years.
Conclusion: The increasing number of CT facilities committing to the use of ISBT 128 is encouraging. Implementing ISBT 128 takes time given the need to update computer systems, or in some cases, to select and implement such systems. While response to the survey was not high, it shows a commitment to implementing both the terminology and the full labeling system which includes electronically-readable key information. Such global implementation will go far towards meeting the WHO recommendation for a globally consistent coding system for human cells.

Expansion of ISBT 128 Terminology to Regenerated and Source Products
Ineke Slaper-Cortenbach, Zbigniew Szczepiorkowski, Matthew Delgado, Paul Ashford and Pat Distler, University Medical Center Utrecht

Aims: In 2006, the International Cellular Therapy Coding and Labeling Advisory Group (CTCLAG) was formed by ICCBBA with support of professional organizations including ISCT. A goal of this group was to standardize terminology used in Cellular Therapy (CT). In 2007, the output of this group was published and thereafter AABB, FACT, JACIE, and NMDP required use of this terminology. New terminology has been developed for regenerated products and source organs used for regenerated products. Once standardized terminology is agreed, assignment of ISBT 128 computer codes follows, supporting bar coding and electronic transmission of information.

Methods: In addition to CTCLAG, the ICCBBA Tissue Engineered Products Technical Advisory Group and Tissue Technical Advisory Groups worked through a consensus process to develop additional terminology. Drafts of terminology were distributed to relevant professional societies for comment and were published on the ICCBBA website for public comment.

Results: CT terminology was updated in 2013 to follow a standard format for the class name: Cell Type, Source (e.g., MNC, Apheresis).

More recently, the class name of regenerated products was agreed to have the format “Regenerated” followed by the tissue type. For example, REGENERATED EPIDERMIS. Additional details such as cell type, delivery mechanism, ancillary substances, excipients, and storage temperature, are added as attributes. An example product description is: REGENERATED EPIDERMIS, from keratinocytes, in the form of a sheet, with ancillary substances present.

The format for class names of organs used as a source of cells for production of cell and tissue products is the organ name followed by the word “Tissue” in parentheses. For example, HEART (Tissue). The word “tissue” was added to distinguish organs intended for transplant from those intended for further processing. Organs for transplant have just the organ name (e.g., HEART).

Conclusion: Through the efforts of experts in the fields of cellular therapy, tissues, and regenerated products working in advisory groups, terminology for medical products of human origin have been developed for CT, tissue, and regenerated products. This terminology is available publicly through the ICCBBA website (www.iccbba.org) and its use for communication and labeling is encouraged.

Validation and implementation of a cost effective electronic document control system.
Zlatibor Velickovic, Janet Macpherson, Craig Wright and John Rasko, Department of Cell & Molecular Therapies, Royal Prince Alfred Hospital

Well organised document control is an essential component of an effective Quality System in a Good Manufacturing Practice (GMP) environment. Manufacturing cell therapies under GMP often generates a large number of controlled documents and records which necessitate complex and laborious management workflows. Electronic document management systems have been available for purchase for over a decade. However the costs associated with the purchase and implementation of such systems have often been seen as prohibitive for small and non-commercial manufacturing facilities.

A cost effective electronic document control system has been introduced to the Australian market recently. Integrated Quality Management System (IQMS) is built on a Microsoft SharePoint platform which provides platform independent document control functionality that can be configured to specific requirements. IQMS provides instant access to documentation and records. It features automated functions for document control such as versioning, reviews, approvals and dedicated storage.
Correct functioning and performance of software and computer systems are essential for obtaining consistency, reliability and accuracy of data in a GMP environment. It is also mandated by the TGA and other regulatory agencies that the implementation and operation of computer systems should be documented and proven to achieve its written objective using a quality system approach.

An IQMS validation protocol was developed using a risk assessment approach and Good Automated Manufacturing Practice (GAMP) guidelines. IQMS is a product with standard interface and functions configured for user specific processes, a category four product based on GAMP guidelines. User requirements were developed and reviewed using a functional risk assessment approach followed by supplier identification, evaluation and audit. The validation protocol was developed to test and verify IQMS functionality and configuration with reference to the approved user requirements. We have demonstrated that the IQMS is an effective and GMP compliant electronic document control system. We recommend the use of electronic document control validation approach based on risk assessment and GAMP guidelines. Detail methodological approach and issues identified during the validation will be discussed.

A83-01, a Small Molecule TGF-beta Receptor Inhibitor Maintains Mesenchymal Stem Cell Phenotype and Function during Serum-Free Culture Expansion
Shanti Gurung, Jerome Werkmeister and Caroline Gargett, CSIRO, VIC
Introduction: Limitations in using mesenchymal stem/stromal cells (MSC) for cell-based therapies includes their spontaneous differentiation to fibroblasts, producing heterogeneous MSC cultures of reduced efficacy. This is an important problem for scale-out production of autologous MSC. The aim of this study was to determine whether A83-01, a TGF-beta receptor inhibitor, prevents differentiation of several MSC types initially purified using the selective perivascular MSC marker, SUSD2, and cultured in serum-free medium (SFM).

Methods: Freshly isolated endometrial and menstrual blood stromal cells were selected by magnetic bead sorting using SUSD2 antibodies and cultured in SFM with bFGF and EGF in 5%O2/5%CO2 until passage 5 (P5). Cultured bone marrow stromal cells and clonogenic placental MSC were similarly selected and cultured a further 2-3 passages in serum and SFM respectively. In the subsequent passage, all cells were incubated with or without A83-01 for 7 days in SFM, then analysed for MSC phenotype, clonogenicity and MSC properties. Flow cytometry was used to quantify autofluorescence, examine cell cycle status and assess apoptosis using Annexin V.

Results: A83-01 dose dependently increased proliferation of P6 endometrial MSC (eMSC) with maximal effect at 1uM. A83-01 increased the %SUSD2+ cells in late passage cultures to >90% for all MSC types (eg eMSC P<0.005 n=9), except placental which increased from 35% to 65%. A83-01-treated cells had higher cloning efficiency (P=0.03 n=9). All A83-01-treated cells expressed MSC surface markers, but eMSC did not express pluripotency markers and retained capacity to differentiate into mesodermal lineages. A83-01-treated P6 eMSCs were increased in the G2/M and reduced in the subG0/G1 phases of the cell cycle (both P<0.02 n=7). Fewer A83-01-treated eMSC expressed Annexin V (P<0.05 n=6) and fewer were autofluorescent (P=0.001 n=14) or stained with β-galactosidase, senescence markers. A83-01-treated SUSD2+ eMSC had reduced phosphorylation of SMAD2/3.

Conclusions: These data suggest that A83-01 promotes SUSD2+ MSC proliferation and blocks senescence and apoptosis in late passage cultures by blocking TGF-beta-mediated apoptosis via signalling through ALK4/5/7 receptors. Small molecules such as A83-01 that promote MSC proliferation and maintains their stemness may enable the expansion of undifferentiated MSC for use in tissue engineering and cell-based therapies for many clinical applications.

Developing a dressing for topical delivery of Multistem® cells to wounds
Giles Kirby, Liesbeth Vandenpoel, Jef Pinxtener, Stuart Mills, Allison Cowin, Rob Short, Andrew Michelmore and Louise Smith, University of South Australia, SA

Translating Cell Therapy 2015
Approximately 450,000 Australians suffer with a chronic wound and it costs the Australian healthcare system approximately $3bn/year. These numbers are set to grow due to an aging population and an increasing incidence of diabetes. These wounds are difficult to treat due to a complex actiology. MultiStem®, multipotent adult progenitor cells, have been shown to be anti-inflammatory, immunomodulatory and are a source of growth factors and cytokines that could help to heal a chronic wound.

Localised delivery of MultiStem® cells to a wound might increase the therapeutic impact of the cells, primarily by confining the cells to the area needed. Plasma polymerisation was used to modify the surfaces of existing wound dressings. These surfaces were optimised to deliver the maximum number of cells from the wound dressing. The ability of the modified wound dressings to deliver cells was assessed using a simple transfer assay whereby the cells were delivered to acellular human dermis. Wound dressings modified with a range of acid or amine functionalities were assessed. Surfaces produced from one of the monomers outperformed the other monomers investigated, delivering 100% of the cells seeded. Surfaces produced according to the gold standard in previous studies with mesenchymal stem cells, acrylic acid, could only deliver 80% of the cells seeded in vitro.

Chimeric Antigen Receptor T Cells as an Immunotherapy for Advanced Melanoma

*Tessa Gargett* and *Michael Brown, Translational Oncology Laboratory, Royal Adelaide Hospital and Centre for Cancer Biology, SA*

Metastatic melanoma has a very poor 5-year survival rate of less than 10% and few treatment options. However melanoma is a promising target for immunotherapy given the existence of an endogenous immune response to melanoma antigens in patients. Potential immunotherapies for melanoma include adoptive cell transfer and the monoclonal antibodies ipilimumab and pembrolizumab, which act to reverse immune blockade and enhance the immune response to melanoma antigens. We have developed chimeric antigen receptor (CAR) T cells for use in the CARPETS Phase I clinical trial, a world-first investigation of CAR T cells in advanced, metastatic melanoma patients currently running at the Royal Adelaide Hospital. The CAR T cells are autologous patient cells that have been genetically engineered ex vivo to express a novel antigen receptor that re-directs T cell specificity towards the tumour-associated antigen GD2. Thus far two patients have been enrolled in the trial at the lowest CAR T cell dose level. Our preliminary results demonstrate that we can consistently generate therapeutic-quality T cells with high levels of GD2-CAR expression, efficient killing of GD2-positive cancer cell lines and short-term persistence following administration. We will also present in vitro data suggest that CAR T cell efficacy may be further enhanced by combining CAR T cells with either BRAF and MEK kinase inhibitors or anti-PD-1 monoclonal antibody.

Live imaging of anti-tumour responses induced by CAR19 T- cells

*Kajal Chaudhry, Ning Xu, Alla Dolnikov and Robert Nordon, Graduate School of Biomedical Engineering, University of New South Wales, Sydney, NSW*

Background and Aims: Targeting cancer cells with chimeric antigen receptor (CAR)-modified T-cells is a novel approach with proven success in early phase clinical trials for leukaemia in adult and paediatric patients. A patient’s own T cells can be genetically modified and amplified in the laboratory to target antigens expressed on tumour cells through the introduction of CAR genes. The cellular mechanisms driving the immune responses mediated by CART-cells remain obscure. While flow cytometry based methods quantify cell killing and conjugate formation at the population level, direct tracking of CART-cell fates by live cell imaging can identify the cues that trigger effector/target cell conjugates and leukaemia cell cytolysis.

Our previous work has shown that CART-cells rapidly differentiate into short-lived effector memory (Tem) cells following antigen-specific stimulation by leukaemia cells. Up-regulation of checkpoint receptor PD-1 accelerating T-cell entry to apoptosis was observed in CART-cells following co-culture with leukaemia cells. Repetitive antigen-specific stimulation from large numbers of target cells promoted effector differentiation and reduced CART-cell numbers, limiting the duration of CART-cell
mediated immune responses. Live cell imaging will be used to understand the cellular mechanism for these observations.

Methodology: Human T cells were genetically modified with CARs targeting CD19 and expressing the CD28 co-stimulatory domain. CD19+ leukaemia NALM6 and Raji cells were co-cultured with CAR or mock T cells at various effector to target(E:T) ratios, with quantification of conjugate frequency, CART-cell division and effector and target cell death using multiparameter flow cytometry. Additionally, conjugate formation and cell lysis were observed directly by live cell imaging on grid arrays (Microsurfaces Pty Ltd). In-house software (MATLAB) was used to track individual cell fates, the dynamics of cell death and conjugate formation.

Results and conclusions: Initial live cell imaging studies have demonstrated that CART cell/target cells conjugation not only induced direct cytolyis of tumour cells but arrested cell division and migration. Thus live cell imaging has elucidated non-cytolytic mechanisms that may play an important role in determining the antileukaemic reactivity of CART-cells.

**Personalised immunotherapy for paediatric cancers**

*Alla Dolnikov and Tracey O'Brien, Sydney Children's Hospital, NSW*

Targeting cancer cells with chimeric antigen receptor (CAR)-modified T-cells is a novel approach with proven success in early phase clinical trials. T-cells modified to express CAR targeting CD19, the antigen expressed in acute lymphoblastic leukaemias (B-ALL), have shown promise as a novel anti-leukaemia therapy. Although the majority of patients responded to CART-cell therapy, variable duration of remission induced by the adoptive transfer of CART-cells was observed. Rapid exhaustion of tumour-targeting T-cells was observed in the patients with the relapsed disease. It was proposed that the exposure to the immunosuppressive tumour microenvironment makes the infused CART-cells dysfunctional. Analysis of tumour biopsies needed to characterise tumour microenvironment might identify signatures that predict susceptibility to CART-cell therapy and define interventions that may be necessary to improve therapeutic efficacy. Using genetic profiling of individual paediatric ALLs we have identified significant differences in the expression of stimulatory and inhibitory immune ligands shaping tumour microenvironment in the bone marrow of ALL patients. Moreover, we and others have shown that modulation of co-stimulatory and/or inhibitory signalling acts to maximise the effect of CART-cells in vitro and in patient xenograft mouse models. Thus the balance between co-stimulatory and inhibitory ligands appears to determine the response of leukaemia cells to CART-cell therapy. We hypothesize that the immune responses mediated by CART-cells against individual ALLs can be leveraged with the modulation of specific immune stimulatory or/and inhibitory ligands identified for each individual patient. Patient tumour sample analysis focused on expression of specific stimulatory and inhibitory immune ligands may help in rationally designing ideal combination immune therapies. High expression of ligands activating immune checkpoint inhibitor receptors expressed on CART-cells validates the use of pharmacological checkpoint inhibitor blockers while low expression of the ligands activating co-stimulatory signalling in CART-cells suggests using the agonists activating co-stimulatory receptors on CART-cells. Incorporation of specific co-stimulatory domains in CAR-structure may also improve CART-cell mediated immune responses in individual ALLs expressing low levels of corresponding co-stimulatory ligand/s. Correlation between the treatment efficacy and the expression of corresponding ligand in individual ALL samples will be determined to identify the biomarkers predicting the efficacy of each immune stimulatory agent.

**Combined immune and epigenetic therapy for high risk and relapsed leukaemias and lymphomas.**

*Alla Dolnikov, Susan Yang, Sylvie Shen, Ning Xu and Tracey O'Brien, Sydney Children's Hospital, NSW*

T-cells genetically modified with Chimeric Antigen Receptor (CAR) to target malignant cells is a novel approach with proven success in early phase human trials. A patient’s own T-cells genetically modified to target tumour associated antigens through the introduction of CAR can be amplified ex-vivo to numbers suitable for adoptive cell therapy and administered to the patient. Engineered T cells
traffic to the bone marrow and kill leukaemia cells. Early phase clinical trials have shown that sustained remission strongly correlates with in vivo CART-cell persistence. We have proposed the use of pharmacological immune modulatory agents to prolong the persistence of CART-cells and improve the efficacy of CART-cell therapy. Multiple studies have revealed that azacytidine (AZA) widely used as a demethylating agent with proven efficacy and limited adverse effects, promotes T-cell immunity. Using xenograft mouse model of chemoresistant paediatric B-cell lymphoblastic leukaemia (B-ALL) we have shown that while neither AZA nor CAR19 T-cells conferred durable responses as a monotherapy in this model, the efficacy of CAR19 T-cell therapy was potentiated by pre-treatment with AZA. These results suggest that AZA and CART-cell combination therapy may be worthy of further consideration for improved treatment of leukaemia. Mechanistically, pre-treatment with AZA produced direct cytotoxic effect on leukaemia cells and indirect effect promoting CART-cell-mediated immune responses. Pre-treatment with AZA increased effector memory T-cell (Tem) numbers in mice infused with CART-cells. AZA pre-treatment of leukaemia cells did not modulate T-cell differentiation but rather expanded Tem cells. AZA pre-treatment did not modulate PD1-expression up-regulated in CART-cells following antigen-specific activation and promoting Tem entry to apoptosis but rather up-regulated the expression of co-stimulatory CD80 ligand activating co-stimulatory CD28 signalling in Tem cells. Gene expression profiling revealed that co-stimulatory ligands are commonly down-regulated in paediatric B-ALL. We propose that AZA treatment given prior to CART-cell infusion may provide a more effective cancer immunotherapy approach compared to CART-cell therapy as a monotherapy.
SPEAKER BIOGRAPHIES

Shelly Heimfeld, Ph.D.
Dr. Heimfeld is a Full Faculty Member at the Fred Hutchinson Cancer Research Center in Seattle, Washington and serves as Scientific Director for the cGTP Cellular Therapy Laboratory and cGMP Therapeutic Manufacturing Facilities. These facilities are responsible for all minimally and more extensively manipulated cell components used for treatment of patients at the Center. His primary responsibilities are to ensure the safety, quality, and effectiveness of each product, but also include implementation of new technologies, translation of basic science procedures into appropriate clinical protocols, product development, process improvement, and regulatory compliance.

Dr. Heimfeld received his Ph.D. in Cell Differentiation from the University of California, Irvine and completed postdoctoral studies with Dr. Irving Weissman at Stanford before going into industry to work as a founding scientist at SyStemix and later at CellPro, the first company to develop an FDA approved device for CD34+ cell enrichment. He is a Past-President of ISCT (International Society for Cellular Therapy), with expertise in FDA regulations and the systems needed for compliance with Good Laboratory Practice (GLP), Good Tissue Practice (GTP), and Good Manufacturing Practice (GMP) in the production clinical cell-based therapies.

Dr. Heimfeld is internationally recognized for his research in hematopoietic-derived stem cells and the development of cell processing technologies for improved cancer therapy. His long-term goals have a direct therapeutic focus, many involving the “engineering” of transplant products to improve clinical outcomes. Current research activities include improvements in isolation/purification technologies for hematopoietic stem/progenitor, T, and B-cell subsets, and development of ex vivo manipulation strategies that can enhance the therapeutic potential of such cells for treatment of various diseases.

Marian STURM PhD, FFSc (RCPA)
Marian Sturm is actively involved in the development and clinical translation of emerging biotherapies, in particular mesenchymal stromal (stem) cells. She has worked for the public health sector for more than 30 years and has extensive experience in both the manufacture of clinical products for transplantation and in research and development. She is Facility Director of the Cell & Tissue Therapies WA (CTTWA) facility at Royal Perth Hospital and Adjunct Associate Professor with the Centre for Cell Therapy & Regenerative Medicine, School of Medicine & Pharmacy and with the School of Pathology & Laboratory Medicine at the University of Western Australia.

She is also a Member of the TGA Advisory Committee on Biologicals. CTTWA is a TGA licensed manufacturing facility, providing an array of clinical products for transplant, including haemopoietic stem cells and human heart valves. It also manufactures mesenchymal stromal (stem) cells under TGA license and has been evaluating these cells in multiple clinical trials for conditions of immune dysregulation in the organ transplant, graft versus host and autoimmune settings and also for tissue repair.

Leighton Clancy, BSc, PhD
Dr Leighton Clancy completed his BSc (Hons) and PhD at the University of New South Wales in medical virology. In 2006 he joined the Sydney Cellular Therapies Laboratory at Westmead Hospital as the production manager of the Immunotherapy program where he has been involved in several trials aimed at addressing infectious complications that arise in patients following transplantation. These trials initially involved the generation of donor derived cytomegalovirus (CMV) specific T cells for the prevention of viral reactivation and have expanded to target several other common post transplant pathogens. Recently he has been involved in establishing a bank of third party virus specific T cells for a multi-centre study to treat antiviral resistant infections. His other interests include T cell immunotherapy targeting leukaemia/tumor associated antigens and the development of clinically acceptable methods to generate chimeric antigen receptor bearing T cells.
Siok Tey, MB BS, FRACP, PhD
I am an NHMRC Early Career Fellow at QIMR Berghofer and a staff specialist in haematology and bone marrow transplantation at the Royal Brisbane and Women’s Hospital. I am a graduate of the University of Queensland medical school and, following physician and pathology training in Brisbane, undertook a research fellowship at the Center for Cell and Gene Therapy at Baylor College of Medicine, Houston, followed by a PhD in immunology at QIMR Berghofer. My research interests are bone marrow transplantation, tumour and viral immunity, and translational immunotherapy.

Allen Chen, PhD
Allen Chen is the Leading Research Scientist of the Stem Cell Bioprocessing Group at Bioprocessing Technology Institute (BTI), A*STAR (Singapore). His research interests focus on the process development for adult and pluripotent stem cell expansion and differentiation using microcarriers. He graduated with honors in bachelor of engineering (bioprocess) in 2001 and received his PhD in biotechnology from University of New South Wales (Australia) in 2006. Afterward, he joined the Bioprocessing Technology Institute (BTI), A*STAR (Singapore) as a research scientist in developing a scalable microcarrier based process for attenuated influenza vaccine production. Later in 2008, he joined the Stem Cell Group within BTI as one of the pioneer scientists in developing scalable bioprocessing platforms for the expansion of human pluripotent stem cells. He has co-authored several publications and reviews in peer reviewed journals and a list of his publications can be found in Singapore Researchers Database (https://researchers.a-star.edu.sg).

Professor Rob Short FTSE
Rob has a long and exemplary track record of research and commercialisation in the fields of plasma, biomaterial science, cell therapy and life science tools research.

He studied Chemistry (BSc) and Physical Chemistry (PhD) at the University of Durham (UK) and joined the University of Sheffield in 1988, where until joining the University of South Australia (UniSA) in 2006, he held the Chair of Material and Biomaterial Chemistry from 2001. During this period, he was a founder of two spin-out companies, CellTran Ltd and Plasso Technology. At UniSA he has held the positions (2006-2015) of Director: Mawson (Research) Institute, Dean: Research of Science and Engineering and Pro Vice Chancellor (Science and Engineering).

Over the past 20 years Rob has published over 180 peer-reviewed articles, won and managed research contracts exceeding AS$100M and venture capital of $16.5M. In 1992 he was awarded the CR Burch Prize (IoP, RSC, UK) for outstanding work in Physical Chemistry for Thin Films or Vacuum. In 2004 he was awarded the UK Society for Biomaterials' second highest prize, the Biocompatibles Endowed Lecture (for mid-career achievement). He has been admitted as a Fellow of the Royal Society of Chemistry and elected to a Fellowship of the Institute for Materials, Minerals, and Mining (IoMMMM) and 2013 was elected a Fellow of the Australian Academy of Technological Sciences and Engineering (FTSE).

Relevant contributions in biomaterial science, in the areas of protein adsorption, cellular attachment and wound-healing led to the development of a bandage that attaches and releases cells into non-healing wounds, such as chronic ulcers. This cell therapy was commercialised in 2000 by the University of Sheffield. Its first product, Myskin™ is used clinically to treat burns, scalds and more recently, chronic ulcers. In 2006 the company merged with a division of Innogenetics, a Belgium multinational, creating a new woundcare company. Myskin™ has been used extensively in UK since 2008 for the treatment of severe burns.

Plasso Technology, an advanced surface engineering company developing tailored coatings for surfaces in diagnostics and life science research was formed in 2003, where Rob, as a founder Director held a position on the Board. In May 2007, the investors realised a successful exit when Plasso Technology was acquired by BD Biosciences, a leading global medical technology company. The BD™ Heparin Binding Plate (as EpranEX is now known) is being used at leading laboratories in Europe and the US to study a wide range of disease processes that involve the heparin family.
Associate Professor Simon Barry BSc, PhD

My lab is interested in how a healthy immune system balances being ready to react by swiftly fighting off pathogens, while maintaining tolerance to harmless challenges such as food and body tissues. My lab is focused on the molecular basis of function and fitness in human regulatory T cells, and the role of the transcription factor FOXP3 in orchestrating this. There is increasing evidence that in a wide number of disease states including autoimmune diseases such as Type 1 diabetes and IBD, Treg cells fail to regulate the immune system effectively, and allow inappropriate destruction of tissues that are essential for life. In order to understand how this breaks down in disease one must first understand what is the basis of a healthy Treg. To do this we use a number of state of the art gene discovery tools such as microarrays and next generation sequencing to identify and then confirm the key genes in Treg function. We were the first to identify the targets of FOXP3 in human Treg using chromatin immunoprecipitation, and we are now developing systems biology approaches to modeling the gene regulation networks in human Treg from healthy and disease samples. Tight regulation of target genes including SATB1 by FOXP3 and microRNAs is a key mechanism by which Treg retain their phenotype, and breakdown of this results in loss of function. Most recently we have established chromatin conformation capture in order to fully map the regulatory interactions in human Treg. A key goal of our research is to fully map the defects resulting in loss of Treg function in disease, so that new approaches to prevent or reverse this can be developed. The lab has also used genome wide approaches to identify putative novel biomarkers of human Treg, and has identified PI16 as a novel T cell surface protein which is expressed on a subset of human Treg. We are characterizing the use of PI16 to isolate human Treg with stable functional characteristics as a tool for Treg cell therapy. In addition, as part of the CRC for cell therapy manufacturing, we are developing functionalized smart surfaces for the clinical expansion of T cells and Treg. These can be delivered to prevent transplant rejection or to reverse the immune destruction of auto immune disease.

Training: After a B.Sc (hons) at Kings College London and Ph.D at Mill Hill, London, Dr Barry undertook postdoctoral training in Adelaide and at the University of Washington in Seattle. He then spent 4 years working as a discovery scientist at Immunex and AMGEN in the US, prior to returning to Adelaide in 2004 to set up a lab.

Lynn O’Donnell, Ph.D.

Dr. O’Donnell received her PhD in Microbiology from the Ohio State University in 1995. She held research positions in the areas of breast cancer and granulocyte differentiation before taking over as Director of the Cell Therapy Laboratory at Ohio State’s James Cancer Hospital and Solove Research Institute in 2000. The Laboratory processes 500 hematopoietic progenitor cell and lymphocyte products annually for the James’ BMT Program and the pediatric BMT Program of Nationwide Children’s Hospital. The Laboratory also performs an additional 1200 other procedures, including sorting cell types for post-transplant chimerism testing, thawing products for infusion and processing investigational cell therapy products for externally sponsored or local investigator-initiated clinical trials. Dr. O’Donnell’s interests include process development of cellular therapies, taking them from research settings to clinical application, and quality assurance and regulatory compliance in cellular therapy. She has served on numerous national and international committees, and currently is ISCT’s Global Secretary, Chair of the Communications Committee, and co-editor of the society’s newsletter, Telegraft.

Jeanette Ripper, PhD

Jeanette began her career as a laboratory scientist at CSIRO in the late 1970’s, working on the development of diagnostic tests for the eradication of diseases from Australian livestock. She was a team member awarded a CSIRO Medal for Research Achievement in 1990, for the developed of a patented platform interferon gamma cellular diagnostic technology (which was commercialised as a rapid test for Mycobacterium tuberculosis & subsequently developed as QuantIFERON®-TB Gold).

Jeanette moved into medical research at the Murdoch Children’s Research Institute in 1992, working on a project investigating the effects of Rotavirus infection on intracellular calcium levels and the mechanisms involved in small intestinal epithelial injury. She transitioned through to cGMP cell therapy manufacturing in 1996, playing a key role in the establishment of the
National (BMDI) Public Cord Blood Bank and leading the team that successfully achieved TGA licensure and FACT accreditation for the bank.

In 2007 Jeanette took up a position as a GMP Inspector at the Therapeutic Goods Administration (TGA), where she performed inspections of both Australian and overseas manufacturer’s, for regulatory compliance for the manufacturing and supply of biologic therapeutic products. As a Lead Inspector, she also represented the TGA in stakeholder engagement, providing quality and regulatory advice in the manufacture of biologics.

Since 2013 Jeanette has been the Quality and Regulatory Manager for Cell Therapies Pty Ltd and its affiliated units at the Peter MacCallum Cancer Centre, where she is responsible for the management and continuous improvement of the Quality System, to ensure GMP compliance across the organisational sites globally for the commercial manufacturing and distribution under license of cellular and tissue therapy products.

Dawn Driscoll, PhD

Dr. Dawn Driscoll is the Principal of DCi Biotech, a consulting firm focused exclusively on the international commercialization of cell, gene and tissue-based therapeutics. DCi Biotech (www.dci-biotech.com) has offices in the US and Australia, to serve global clients. Dr Driscoll’s role at DCi is to advise on regenerative medicine business strategy and to conduct due diligence, primary market research, advisory boards, reimbursement strategy, and global financial forecasting. Prior to DCi, Dawn worked for Johnson & Johnson in global Business Development roles, focused primarily on biotechnology licensing, mergers and acquisitions.

Sue O'Malley PhD

Dr O’Malley is the principal consultant and founder of Medical Intelligence, an independent consulting business specialising in all areas of Australian health technology assessment (HTA) excluding pharmaceuticals. In recent years Medical intelligence has become one of the few specialist consultancies covering reimbursement for surgical, diagnostic and pathology procedures, medical devices, and prostheses. Sue submitted her first application to the Medical Services Advisory Committee (MSAC) for Medicare Benefits Schedule (MBS) funding in 1999 and by 2015 has been involved in more applications than any other individual (excluding Department of Health Contracted Assessors). She has also submitted numerous applications to the Prostheses List Advisory Committee (PLAC) for the funding of a wide variety of prostheses for listing on the Prostheses List. Medical Intelligence also provides services covering financial and economic modelling and strategic analysis of technologies that are not funded via the MBS or the Prostheses List. Included in its clientele are a number of Australian companies as well as many subsidiaries of multinationals.

Dr O’Malley started as a Health Economist in 1993 as Principal Economist for Northern Sydney Area Health, NSW State Government. This appointment followed several years working as a senior public servant working as both an Economist and a Policy Coordinator. Since starting in the Health Industry she has worked in Biotechnology, Pharmaceutical, and Medical Devices companies. She holds a Master’s Degree in Public Economics and in 2011 was awarded a Doctorate of Philosophy based on a thesis by publication titled “Funding issues of new and emerging medical technology in Australia”.

Between July 2012 and July 2015 Dr O’Malley held the part-time position of Associate Professor of Health Economics, Faculty of Medicine and Health Sciences, Macquarie University.

Dr O’Malley has published widely on the Australian Medical Services Advisory Committee (MSAC) and related Health Economics topics. She is a reviewer for the MJA and BMJ.

Dr O’Malley was a board member of the Association of Regulatory and Clinical Scientists (ARCS) up to the end of 2008 and is a past chair of the ARCS Health Economics and Medical Devices Education Sub-Committees. She currently sits on the Medical Technologies Association of Australia (MTAA) Reimbursement Sub-Committee. Since 2010 she has been the principle organised of the Medical Technology Funding stream of the annual ARCS Canberra Congress. She also runs training course on the MSAC and Prostheses List Application processes for the MTAA.

In 2015 Dr O’Malley accepted an invitation to be a member of MSAC’s Evaluation Sub-Committee (ESC).

Michael H. May PhD.

President & CEO Centre for Commercialization of Regenerative Medicine

Dr. May completed his PhD in Chemical Engineering at the University of Toronto in 1998 as an NSERC Scholar and was awarded the Martin Walmsley Fellowship for Technological Entrepreneurship.
Michael May is currently President and Chief Executive Officer of the Centre for Commercialization of Regenerative Medicine (CCRM), a Canadian, federally incorporated, not-for-profit organization dedicated to supporting the development of foundational technologies that accelerate the commercialization of stem cell- and biomaterials-based technologies. Prior to CCRM, Michael was the President, and co-founder, of Rimon Therapeutics Ltd., a Toronto-based regenerative medicine company developing novel medical polymers that possess drug-like activity.

Michael sits on a number of Boards and advisory committees including: MaRS Innovation; 20/20 Vision - a centre of excellence for biomaterials in ophthalmology; the Ontario Institute of Regenerative Medicine (OIRM); the Centre for the Commercialization of Antibodies and Biologies (CCAB); the Department of Chemical Engineering and Applied Chemistry at the University of Toronto; the Center for Diseases of Aging within the Vaccine & Gene Therapy Institute of Florida (VGTI) and Karolinska Institutet of Stockholm, Sweden; the Industry Committee of the International Society for Stem Cell Research (ISSCR); the Executive Committee, Alliance for Regenerative Medicine (ARM); ExCellThera Ltd.; the Rick Hansen Institute Commercialization Program Advisory Committee; Toronto Region Board of Trade Life Sciences Steering Committee; the Biozone Commercialization Committee; the NSERC CREATE M3 Program Committee; and the Cell and Gene Therapy Insights, Editorial Advisory Board.

William Mercer

Mr. Bill Mercer is a Canadian citizen who graduated from the University of Western Ontario with a BSc of Chemistry. He recently retired after 28 years with Terumo BCT (COBE, Gambro and CaridianBCT). He is truly a global business executive having achieved success on the world stage. The majority of his efforts have been concentrated in the medical device field. His expertise is in business strategy, new product/process development, commercialisation and organisational effectiveness. He also held a non executive director’s position with TransBio (CRC for Biomarker Translation).

His focus has been on two fronts; cell based therapeutics of an immunological, haematological and regenerative medicine nature and emerging market development in China and South East Asia. The coupling of well-structured project plans, defined scientific investigation, thorough market research, timely review with effective metrics plus superior communication has helped deliver superior outcomes. Tied with a very process mindset and a worldwide network, his passion is to deliver superior patient value within a commercial context. He has lived in Canada, Australia, United States of America and in Hong Kong while working for Terumo BCT.

Dr Sherry Kothari, CRC for cell manufacture

Dr Sherry Kothari is the Chief Executive and Managing Director of the CRC for Cell Therapy Manufacturing. The CRC for Cell Therapy Manufacturing is focused on the cost-effective manufacture and rapid translation of cell therapies into clinical practice. The CRC will provide new treatments and develop new materials-based manufacturing technologies for the treatment of conditions such as diabetes, chronic wounds, cardiovascular disease, and immune-mediated diseases such as graft versus host disease. As well as her appointment at the CRC, Sherry has extensive experience in working within bioscience at the research/industry/clinical interface. Sherry started her career in maxillofacial surgery before undertaking a PhD at the University of Sheffield, focusing on healing at the bone implant interface. After lecturing in Medical Materials and Tissue Engineering at the University of Manchester, she undertook an Executive MBA, to build on her passion for entrepreneurship and translation within the biotechnology sector. Sherry has been a founder and investor in two successful university life sciences spinout companies and has since played a significant role in introducing an entrepreneurial culture within science and engineering in higher education, including helping mentor and fund early start-up ventures. Sherry has been on the Board of SIFE (Students in Free Enterprise), raising the capacity for invention, innovation, commercialisation, technology acquisition and new business growth. Sherry moved to Australia in 2007. Based at the University of South Australia, she established partnerships across Australia, Asia and Europe. In 2011, she took over as Program Leader for one of the three research programs of the Wound Management Innovation CRC, before taking on her current role at the CRC for Cell Therapy Manufacturing.
TESTING
NRL provides TGA-licensed and TCO 88 compliant testing for infectious diseases including HIV and hepatitis B and C using both serological and nucleic acid testing methods. NRL also provides reference testing for HIV, HCV and HTLV using testing strategies that include selected tests not widely used by other laboratories.

<table>
<thead>
<tr>
<th>TYPE OF TESTING</th>
<th>REFERENCE TESTING</th>
<th>TGA LICENSED TESTING</th>
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<tbody>
<tr>
<td>HIV Serology</td>
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<tr>
<td>HCV Serology</td>
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<td>HBV Serology</td>
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<td>HTLV Serology</td>
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<td>Syphilis Serology</td>
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<tr>
<td>HIV-1 RNA</td>
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<tr>
<td>HCV RNA</td>
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<td>HGV DNA</td>
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<tr>
<td>HTLV-1 Proviral Load</td>
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</tbody>
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Subject to further consultation with NRL

QUALITY ASSURANCE
NRL offers the complete package of quality assurance solutions to ensure accuracy and precision of tests and testing processes. External Quality Assessment Schemes, Quality Control and Specificity Monitoring programmes are provided for a range of infectious diseases.

ACCREDITATION/ CERTIFICATION
Accredited by NATA as a Medical Testing Laboratory, compliant with ISO/IEC 15189: 2013

Accredited by NATA as a Proficiency Testing Scheme Provider, compliant with ISO 17043: 2010

Licensed by TGA to the Code of Good Manufacturing Practice for human blood and blood components, human tissues and human cellular therapy products (2013) and as compliant with TGO 88.

In 1985, NRL (based in Melbourne) was established as Australia’s National HIV Reference Laboratory and since then has expanded its expertise to provide an array of services. These include the provision of laboratory quality assurance programmes, pre-market assessments of in vitro diagnostic medical devices, specialised and reference testing and the delivery of training programmes for strengthening laboratory systems.

Tel: +61 3 9418 1111 □ Email: info@nrl.gov.au
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Joey Tan
Sales and Marketing Manager
ams Laboratories Pty Ltd
Sydney office: 8 Rachael Close, Silverwater NSW 2128 Australia
Brisbane office: 2/120 Bluestone Circuit, Seventeen Mile Rocks QLD 4073 Australia
Tel:+61 2 9704 2300 | Mobile:+61 478 001 570 | Email:joey@amslabs.com.au
General Enquiry: info@amslabs.com.au | Website:www.amslabs.com.au

**BD**

is a leading global medical technology company that develops, manufactures and sells medical devices, instrument systems and reagents.

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Founded in 1897 and headquartered in Franklin Lakes, New Jersey, BD employs nearly 45,000 associates in more than 50 countries throughout the world. The Company serves healthcare institutions, life science researchers, clinical laboratories, the pharmaceutical industry and the general public.

For more information, please visit www.bd.com/anz

**Cell Therapies**
is the leading manufacturer and distributor of cellular therapies in Asia Pacific, a region of over 650 million people. Cell Therapies provides the essential manufacturing and distribution infrastructure for the safe, reliable and scalable delivery of cellular therapies into clinical trials and for therapeutic use.

For over a decade, Cell Therapies has made these capabilities available on a contract basis to some of the world’s leading stem cell companies, developing products and processes that are compliant with global regulatory and cGMP requirements and successfully integrating with global manufacturing networks. Cell Therapies also in-licenses late stage products for high unmet clinical need for commercialisation in SE Asia and Australasia.

Cell Therapies Pty Ltd
Ground Floor, 10 St Andrews Place
East Melbourne VIC 3002 Australia
Phone: +61 3 9656 5804
Tim Oldham TimOldham@celltherapies.com.au Mobile: +61 (0)403 446 665
Dominic Wall DominicWall@celltherapies.com.au Mobile: +61 (0)417 031 356
Sven Straub SvenStraub@celltherapies.com.au Mobile: +61 (0)404 830 310

Translating Cell Therapy 2015
Fresenius Kabi Australia Pty Limited is part of the Fresenius Kabi Group, a global healthcare company that specialises in lifesaving medicines and technologies for infusion, transfusion and clinical nutrition. With our corporate philosophy of “caring for life”, we are dedicated to the needs of critically and chronically ill patients by delivering high quality, effective medicines and technology.

Fresenius Kabi Australia Pty Limited specialises in:
- Generic Drugs
- I.V Fluids & Irrigation Solutions
- Gastroenterology
- Parenteral Nutrition
- Infusion and Transfusion Technology
- Devices for Cell Therapy Research

Fresenius Kabi Australia Pty Limited
Customer Service Ph 1300 732 001
Customer Service Fax 1300 304 384
Website www.fresenius-kabi.com.au
Email customer.service-au@fresenius-kabi.com

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Xuri products deliver integrated solutions to help make cellular therapies a clinical reality, supporting the transition from research to clinics with confidence.

Xuri - Believe in better futures
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- Accelerating breakthrough cell therapies
- Addressing healthcare’s toughest challenges

Jon Ince BSc MBA
BioProcess Strategic Account Manager
GE Healthcare
Life Sciences ANZ
M +61 404 027 237
T +612 9846 4964
jon.ince@ge.com

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SCIENTIFIC is the world leader in serving science. Our mission is to enable our customers to make the world healthier, cleaner and safer. Through our Applied BioSystems, Invitrogen and Gibco brands, we provide a complete portfolio of products and services to support every step of stem cell research.

Naomi Morison, Ph.D
Technical Sales Specialist
Primary/Stem Cell Culture, Transfection and Drug Discovery
Life Sciences Solutions
Thermo Fisher Scientific
5 Caribbean Dv, Scoresby Vic 3179
T +61 411 565 128
naomi.morison@thermofisher.com
www.lifetechnologies.com.au

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Celebrating its 20th Anniversary, ICCBBA is the not-for-profit, nongovernmental international standards organization in official relations with the World Health Organization responsible for the management and development of the ISBT 128 Standard. Used in more than 75 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.

Matt Delgado
Company ph +1-909-793-6516
Email: iccbba@iccbba.org

For a quarter century, Miltenyi Biotec has helped brilliant minds advance scientific discovery and cellular therapies. On our 25th anniversary, we look back at the milestones that got us where we are today, and the breakthroughs that inspire us for tomorrow.

Our commitment is to the advancement of biomedical research and cellular therapy. Our passion is the development of technologies that impact on basic research and clinical applications. Our customers value our reputation and years of experience in providing products that make a difference.

Miltenyi Biotec Australia Pty. Ltd.
Unit 16 A, 2 Eden Park Drive
Macquarie Park NSW 2113 Australia
Phone: +61 2 8877 7400
Fax: +61 2 9889 5044
E-Mail: macs@miltenyibiotec.com.au
Web: www.miltenyibiotec.com
Technical support E-Mail: macs@miltenyibiotec.com.au

Miltenyi Biotec

NextCell Pty Ltd provides small scale GMP compliant manufacturing capabilities designed to translate research into early stage clinical trials. Specialising in processes involving human cells and tissue, we apply full Quality Assurance practices throughout to ensure complete compliance. NextCell operate out of state-of-the-art facilities based at the University of South Australia’s Mawson Lakes Campus, comprising of two Class B cleanrooms and full supporting infrastructure.

Contact: info@nextcell.com.au

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In 1985, NRL was established as Australia’s National HIV Reference Laboratory. Since then, it has expanded its expertise to provide a range of laboratory related services. These include specialised and reference testing, the provision of laboratory quality assurance programmes and pre-market assessments of in vitro diagnostic medical devices (IVD) assessments, which are conducted under contract to the Therapeutic Goods Administration (TGA) and to the World Health Organization.

NRL provides TGA-licensed and TGO 88 compliant testing for infectious diseases (including HIV and hepatitis B and C) by serology and nucleic acid testing of samples collected from blood and tissue donors. NRL also provides reference testing for HIV, HCV and HTLV, applying testing strategies that include IVDs not widely used by other laboratories.

For more information about any of these services please contact NRL at: info@nrl.gov.au or visit the NRL website: www.nrl.gov.au

Phillips Ormonde Fitzpatrick (POF) offers a comprehensive range of services to help clients identify, protect and enforce their intellectual property assets, including patents, trade marks, designs and copyright. POF has a dedicated Chemistry and Life Sciences team, comprising of professionals with specialist qualifications and experience in biotechnology, chemistry and pharmaceuticals, with a strong focus on medical technology and research. Our client base includes global biotechnology and pharmaceutical corporations, world renowned universities, leading research institutes and multinational corporations.

Phillips Ormonde Fitzpatrick
Level 5/75 Hindmarsh Square Adelaide South Australia 5000 Australia
T: +61 8 8232 5199
www.pof.com.au
DELEGATES

Vicki Antonenas
Zafar Anwar
Bob Atwill
Marco Baccanti
Simon Barry
Sue Best
Emily Blyth
Leon Brownrigg
Kajal Chaudhry
Allen Chen
Leighton Clancy
Tongted Das
Matt Delgado
Justin Dibbens
Ala Dolnikov
Dawn Driscoll
Pamela Dyson
David Facey
James Favaloro
Dominic Fernandez
Tessa Gargett
Caroline Gargett
David Gottlieb

Fran Harding
Uwe Eduard Hattenhorst
Shelly Heimfeld
Jon Ince
Kilian Kelly
Sherry Kothari
Charlie Latham
Gail Lazzaro
Stephen Livesey
Sue Low
Janet Macpherson
adel malek
Michael May
Andrew McCutchan
William Mercer
Nancy Messino
Guy Moeneclaey
Naomi Morrison
Ayse Mouminoglu
Tanja Mueller
Robert Nordon
Eve O'Connor
Lynn O'Donnell

Sue O'Malley
Leanne Purins
Scott Ragg
John EJ Rasko
Jeanette Ripper
Hugh Salisbury
Rob Short
Tony Simula
Louise Smith
Nathan Smith
Paula Stoddart
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