

PROGRAM

Wednesday 24th May 2017

Registration

8:00 - 5:30

Pre-Function

Foyer

Welcome and Opening

AGCTS and ASSCR Presidents

Executive Director OHMR (Antonia Penna)

9:00 – 9.30

Joint Society Session 1: Applications at the Interface of Cell & Gene Therapy

Chair: Rose Martiniello-Wilks

9:30 – 11:00

- A/Prof. Alexandra Sharland, Centenary Institute, Australia. *Liver-directed gene transfer for transplantation tolerance induction.* [30 minutes]
- Dr. Michael O'Connor, Western Sydney University, Australia. *Pluripotent stem cell derived lenses model drug induced cataract.* [30 minutes]
- Prof. Eva Feldman, University of Michigan, USA. *A comparison of clinical outcomes between two open label FDA approved trials of intraspinal stem cell transplantation of ALS patients and available historical ALS databases.* [15 minutes]
- Dr. Kenneth Hsu, Kid's Research Institute, Australia. *Developing CAR T cells to target paediatric solid tumours.* [15 minutes]

Morning Tea, Trade Display & Poster Viewing

11:00 – 11.30

Concurrent ASSCR Session 1: Hematopoietic Stem Cell Differentiation & Mobilisation

Chair: Ed Stanley

11:30 – 13:15

- Prof. Susie Nilsson, CSIRO, Australia. *Optimising a mobilised blood cell product.* [30 minutes]
- Dr. Elizabeth Ng, Murdoch Children's Research Institute, Australia. *Generation of definitive haematopoietic lineages from human pluripotent stem cells.* [30 minutes]
- Prof. Kiarash Khosrotehrani, University of Queensland, Australia. *Functional definition of endothelial hierarchy from progenitor to mature endothelial cells in adult vasculature.* [15 minutes]

- Jane Li, University of New South Wales, Australia. *Lab-on-a-chip simulation of embryonic blood flow to study the mechanobiology of endothelial to haematopoietic transition.* [8 minutes]
- Mr. Ali Motazedian, Murdoch Children's Research Institute, Australia. *"Stromal free" T-cell development from pluripotent stem cells.* [8 minutes]

Concurrent AGCTS Session 1: Vector Development

Chair: Paul Gregorevic

11:30 – 13:15

- Dr. Leszek Lisowski, Children's Medical Research Institute, Australia and Military Institute of Hygiene and Epidemiology (MIHiE), Puławy, Poland. *The many faces of AAV: from gene addition tool, through gene editing platform to unexplored evolution.* [30 minutes]
- Prof. Uta Griesenbach, Imperial College London, UK. *The ups and downs of CF gene therapy.* [30 minutes]
- Dr. Samantha Ginn (AGCTS Esteemed Member Award) Children's Medical Research Institute, Australia. *Development of recombinant adeno-associated virus (rAAV) vectors for genome editing of murine hepatocytes containing a human target sequence for repair by homologous recombination.* [15 minutes]
- Dr. Rakesh Veedu, Murdoch University, Australia. *Evaluation of exon skipping using novel chemically-modified nucleic acid analogues.* [15 minutes]
- Prof. Ian Alexander, Children's Medical Research Institute, Australia. *Discovery of a liver-specific enhancer-promoter element in the 3'UTR of the wild-type AAV2 genome provides novel insights into AAV vector safety in the human liver.* [15 minutes]

Lunch & Trade Display Viewing

13:15 – 14:00

Plenary Session #1: Regenerative Medicine & Bioengineering

Chair: Michael Doran

14:00 - 15:30

- Dr. Kimberly Homan, Wyss Institute, USA. *Bioprinting Living Tissue.* [45 minutes]
- A/Prof. Enzo Porrello, Murdoch Children's Research Institute, Australia. *Re-activating the neonatal proliferative network for heart regeneration.* [45 minutes]

Afternoon Tea, Trade Display & Poster Viewing

15:30 - 15:45

Concurrent AGCTS Session #2: Disease Frontiers for Gene Therapy

Chair: Jim Vadolas

16:00 - 17:30

- Dr. Masatoshi Tagawa, Chiba Cancer Center, Japan. *Preclinical and clinical research for malignant mesothelioma with gene medicine and bisphosphonates.* [30 minutes]
- Prof. Steve Wilton, Murdoch University, Australia. *Redirecting pre-mRNA splicing as a therapy for Duchenne muscular dystrophy.* [30 minutes]
- Dr. Georg von Jonquieres, University of New South Wales, Australia. *Novel approaches to prevent the NAAsty - Targeted gene therapy for leukodystrophies.* [15 minutes]
- Dr. May Aung-Htut, Murdoch University, Australia. *Late onset Pompe disease: rescue of acid alpha-glucosidase expression by splice modification.* [15 minutes]

Concurrent ASSCR Session #2: Systems Biology & Epigenetics

Chair Helen Abud

16:00 – 17:30

- Prof. Christine Wells, University of Melbourne, Australia. *The systems biology of stem cells.* [30 minutes]
- Dr. Partha Das, Monash University, Australia. *Role of epigenetic regulators in embryonic stem cells.* [30 minutes]
- Dr. Jessica Vanslambrouck, Murdoch Children's Research Institute, Australia. *Piggybac transposon-mediated direct transcriptional reprogramming to nephron progenitors.* [15 minutes]
- A/Prof. Bronwen Connor, University of Auckland, New Zealand. *Enhanced conversion of adult human fibroblasts into induced neural precursor cells using chemically modified mRNA.* [15 minutes]

Welcome Reception, Poster Session/Judging

17:30 – 19:00

Thursday 25th May 2017

Corporate Breakfast Session

7:30am - 8:15am

[Corporate Speaker: Miltenyi] Prof. Richard Boyd, Australia. *Clinical translation of CAR T and iPS cell therapies: How to bridge the gap.* [30 minutes]

Registration

8:00 - 5:30

Pre-Function

Foyer

Plenary Session #2: Clinical Translation of Cell & Gene Therapy

Chair: Steve Wilton

8:45 - 10:30

- Ms Anne O'Neill, Office for Health & Medical Research, NSW Health, Australia. *OHMR Medical Device support program.* [15 minutes]
- Dr. Christine Chio, Cold Spring Harbour, USA. *Organoids to study pancreatic cancer.* [45 minutes]
- Prof. Steve Petrou, The Florey Institute of Neuroscience & Mental Health, Australia. *Epilepsy in a dish: Disease mechanism based therapies in neurogenetic disorders.* [45 minutes]

Morning Tea, Trade Display & Poster Viewing

10:30 - 11:00

Concurrent ASSCR Session #3: Disease Modelling and Mechanisms

Chair: Melissa Little

11:15 - 13:00

- A/Prof. Julian Heng, University of Western Australia, Australia. *Novel molecular mechanisms which underlie neural stem cell dysfunction in human brain developmental disorder.* [30 minutes]
- A/Prof. Helen Abud, Monash Biomedicine Discovery Institute, Australia. *Stem cell and niche interactions in the intestinal epithelium.* [30 minutes]
- Dr. Eva Tomaskovic-Crook, University of Wollongong, Australia. *Building brain-organoids through 3D bioprinting: Towards 3D Modelling Of Neural Development, Disease and Regenerative Medicine.* [15 minutes]
- Dr. Dmitry Ovchinnikov, University of Queensland, Australia. *Manipulation of the APP levels using CRISPR/Cas9-based technologies in a Down syndrome iPS-based in vitro neurogenesis model reveals its substantial contribution to Alzheimer disease-like neuropathology.* [15 minutes]

- Ms Freya F Bruveris, Murdoch Children's Research Institute, Australia. *RUNX1 signaling, but not SOX17 is required for the formation of human haemogenic endothelium derived primitive blood cells.* [15 minutes]

Concurrent AGCTS Session #3: Clinical Trials Using Gene Therapy

Chair: Ian Alexander

11:15 - 13:00

- Dr. Nick Smith, Women's and Children's Hospital, Adelaide, Australia. *Gene therapies in paediatric neurodegenerative disease: Clinical trials in Sanfilippo syndrome.* [30 minutes]
- Prof. Elizabeth Rakoczy, Lions Eye Institute, Australia. *One year safety and patient focused post hoc efficacy analysis of gene therapy using rAAV.sFlt-1 for wet Age Related Macular Degeneration (wet AMD).* [30 minutes]
- Dr. David Suhy, Benitec, USA. *Towards development of a "silence and replace" based approach for the treatment of Oculopharyngeal Muscular Dystrophy.* [15 minutes]
- Dr. Belinda Kramer, Kid's Research Institute, Australia. *Outcomes of a Phase I trial for Cancer Gene Therapy in paediatric patients.* [15 minutes]
- Dr. Jim Hu, University of Toronto, Canada. *Stem/progenitor cells targeting in airway for lung gene therapy with helper-dependent adenoviral vector.* [15 minutes]

Lunch & Trade Display Viewing

13:00 - 14:00

AGCTS Annual General Meeting

14:00 - 15:00

ASSCR Annual General Meeting

14:00 - 15:00

Afternoon Tea, Trade Display & Poster Viewing

15:00 - 15:20

Joint Plenary Session #3: Disease Modelling & Drug Screening

Chair Michael O'Connor

15:20 - 17:00

- [Corporate Speaker: Illumina] Ralf Moser, *A scalable high-throughput method for RNA-Seq analysis of thousands of individual cells.* [10 minutes]
- Prof. Amit Nathwani, University College London, UK. *Progress with Haemophilia Gene Therapy.* [45 minutes]

- A/Prof. Brian Kaspar, Nationwide Children's Hospital, USA. *Gene Transfer and Translation in Neuromuscular Disease*. [45 minutes]

Poster Session/Judging

17:00 - 18:00

Joint Conference Dinner

19:00 - 22:00

Friday 26th May 2017

Registration

8:00 - 10:00

Foyer

Pre-Function

Plenary Session #4: New Developments in Vector Design & Gene Editing

Chair: Paul Gregorevic

9:00 - 10:30

- Dr. Luk H. Vandenberghe, Grousbeck Gene Therapy Center, Mass Eye and Ear, Harvard, USA. *Guided by evolution: Gene transfer biology and AAV discovery.* [45 minutes]
- Dr. Sara Howden, Murdoch Children's Research Institute, Australia. *The induced pluripotency and genome editing revolution.* [45 minutes]

Morning Tea, Trade Display & Poster Viewing

10:30 - 11:00

Concurrent AGCTS Session #4: Cell & Gene Therapy Strategies

Chair: Ann Simpson

11:00 - 12:30

- Dr. Eddy Kizana, Westmead Institute for Medical Research, Australia. *Gene therapy for cardiac arrhythmias.* [30 minutes]
- Dr. Ken Micklethwaite, Westmead Institute for Medical Research, Australia. *Transposon tools for CAR T-cells.* [30 minutes]
- Dr. Chandana Herath (AGCTS Esteemed Member Award) University of Melbourne, Australia. *A single treatment with ACE2 using AAV2/8 vector inhibits liver fibrosis associated with fatty liver and biliary diseases in mice.* [15 minutes]
- Dr. Jim Vadolas, Murdoch Children's Research Institute, Australia. *Harnessing intronic delivery of functional shRNAs for gene therapy.* [15 minutes]

Concurrent ASSCR Session #4: Regenerative Medicine & Cell Therapy

Chair: Michael Doran

11:00 - 12:30

- A/Prof. Clare Parish, The Florey Institute of Neuroscience & Mental Health, Australia. *Utilizing biomaterials and human pluripotent stem cell to repair the injured brain.* [30 minutes]
- Prof. Peter Currie, Australian Regenerative Medicine Institute, Monash University, Australia. *Muscle stem cells in growth and regeneration.* [30 minutes]

- Dr. Thierry Jarde, Monash University, Australia. *Neuregulin1 / ErbB signalling regulates intestinal stem cells during tissue homeostasis and regeneration.* [15 minutes]
- Ms. Shuang Cui, Flinders University, Australia. *Wnt regulates muscle stem cell differentiation require β -catenin.* [8 minutes]

Lunch & Trade Display Viewing

12:30 – 13.30

ASSCR Junior Investigator Event

Chair: Julian Heng

13:30 – 14:30

[8 minutes, plus 2 minutes of questions]

- Dr. Tom Forbes (PhD student) Murdoch Children's Research Institute, Australia. *Gene correction of IFT140 mutation rescues ciliary phenotype in patient iPSC-derived kidney organoids.*
- Mr. Jaber Firas (PhD student) Monash University, Australia. *Short induction of the Yamanaka factors or Klf4 in fibroblasts gives rise to a cell population capable of transdifferentiation without traversing through a pluripotent stage.*
- Ms Hananeh Fonoudi (PhD student) Victor Chang Cardiac Research Institute & University of New South Wales, Australia. *Routes to better understand genetic causation of hypoplastic left heart.*
- Dr. Benjamin Cao (ASSCR ECR Award applicant) CSIRO, Australia. *Chemosensitisation of acute lymphoblastic leukaemia using a small molecule integrin antagonist.*
- Dr. Jennifer Durnall (ASSCR ECR Award applicant) The Florey Institute of Neuroscience & Mental Health, Australia. *Novel human GAPTrap hPSC lines for tractable and modulatory in vivo analysis in rodents.*
- Dr. Thao Nguyen (ASSCR ECR Award applicant) University of Adelaide, Australia. *EphA forward signalling enhance human haematopoietic progenitor cell migration and adhesion via Rac1 activation.*

AGCTS Junior Investigator Event, Chair: Samantha Ginn

13:30 – 14:30

[8 minutes, plus 2 minutes of questions]

(All eligible for AGCTS Student Member Oral Prize)

- Ms Que La (PhD student, AGCTS Panos Iannou Award applicant) University of Technology Sydney, Australia. *Expression of human pancreatic transcription factors in the livers of FRG mice.*
- Mr Craig McIntosh (PhD student) Murdoch University, Australia. *The use of antisense oligonucleotide-mediated exon skipping to treat spinocerebellar ataxia type 3.*
- Miss Hannah Pearce (PhD student, AGCTS Pano Iannou Award applicant) Monash University, Australia. *Re-targeting rAAV6 towards inflamed endothelial cells.*
- Mr Bao Le (PhD student, AGCTS Panos Iannou Award applicant) Murdoch University, Australia. *Exploring novel therapeutic chemistries in exon-skipping for Duchenne muscular dystrophy.*
- Ms Anais Amaya (PhD student, AGCTS Panos Iannou Award applicant) Children's Medical Research Institute, Australia. *Treatment of genetic liver disease by AAV-mediated genome editing and selective expansion of repaired hepatocytes.*
- Mr Marti Cabanes Creus (PhD student) Children's Medical Research Institute, Australia. *Selection of novel replication competent AAV capsid libraries leads to an unexpected outcome in primary human CD34+ and iPSC-derived neuron cells.*

Joint Society Session 2: First-in-Man Trials: twists and turns on the road to translation

Chair: Megan Munsie

14:30 - 16:00

Panel:

Amit Nathwari, UCL, UK

Eva Feldman, University of Michigan, US

Megan Donnell, Sanfilippo Children's Foundation, Australia

Christopher Scott, Baylor College of Medicine, US

Janet Macpherson, Royal Prince Alfred Hospital, Australia

Best Poster/Presentation Awards and Closing Remarks

AGCTS and ASSCR Presidents

16:00 - 16:30