Australian Academy of Science

THEO MURPHY HIGH FLYERS **THINK TANK 2015**

# THE STEM CELL REVOLUTION

## Lessons and imperatives for Australia

***PROGRAM***

*22–24 July 2015, The Menzies Hotel, Sydney*

# CONTENTS



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**#TMThinkTank**

# FOREWORD

The Academy has been hosting annual High Flyers Think Tanks on nationally important topics since 2002. The purpose of these events is to bring together outstanding early- and mid-career researchers with expertise in a broad range of disciplines to engage in discussing novel applications of science and technology, and to identify gaps in knowledge that need to be addressed.

This year, the Academy is inviting 60 of the brightest early- and mid-career researchers from around Australia and neighbouring countries to take stock of scientific developments in the stem cell science field and consider the political, regulatory and ethical issues in their broadest context. Internationally-renowned senior scientists will also provide insights into current perspectives in the field.

The Think Tank participants will aim to identify creative solutions to issues and consider policy options across four key areas:

- Platform science (across disease modelling, genomics, IPS, etc)
- Clinical translation (Australian perspectives, including infrastructure considerations)
- Public perspectives and expectations; Political and regulatory landscape
- Current and future directions for stem cell science.

For the participants, the Think Tank will be an outstanding opportunity to develop expertise in operating in a multi-disciplinary context and in understanding the contribution of science to evidence-based policy formulation. Further, the event will be a unique networking and career development opportunity for the nation's next generation of science leaders.

Following the event, the findings of the Think Tank will be published in a recommendations report which will be made available to government, stakeholders, interested parties and the public. Reports from previous Think Tanks have been timely, well received and instrumental in influencing policy development.

The 2015 Think Tank is generously supported by the Theo Murphy (Australia) Fund, which is administered by the UK Royal Society. The Academy is delighted to have this funding available to enable some of Australia's brightest young scientists to engage in fresh thinking about a fundamental issue for our nation's future, and to develop networks that will enrich their careers.

**Professor Andrew Holmes** AM PresAA FRS FTSE
President, Australian Academy of Science

# PROGRAM OF EVENTS

## *Day 1* Wednesday 22 July

| | |
|---|---|
| 6.30 pm | Registration |
| **Session 1: Opening session—Sydney Room** | |
| 7.00 pm | **Welcome**<br>Professor Richard Harvey |
| 7.05 pm | **Opening address**<br>The Hon Sarah Mitchell MLC,<br>NSW Parliamentary Secretary<br>for Regional and Rural Health |
| 7.20 pm | **Reception and social gathering** |
| 9.30 pm | **End of Session 1** |

## *Day 2* Thursday 23 July

| | |
|---|---|
| 8.30 am | Registration |
| **Session 2: Presentations—Sydney Room** | |
| Chaired by Professor Richard Harvey and Professor Martin Pera | |
| 9.00 am | **Introduction**<br>Professor Richard Harvey |
| 9.05 am | *Lgr5 stem cells and organoids in health and disease*<br>Professor Hans Clevers, Hubrecht Institute<br>25 MIN PRESENTATION + 10 MIN DISCUSSION |
| 9.40 am | *Exploring the boundaries of transcription factor-mediated reprogramming*<br>Associate Professor Jose Polo, Monash University<br>25 MIN PRESENTATION + 10 MIN DISCUSSION |
| 10.15 am | Morning tea |
| 10.45 am | *From concept to clinic: challenges in the translation of cellular therapies*<br>Professor Paul Simmons, Mesoblast<br>25 MIN PRESENTATION + 10 MIN DISCUSSION |
| 11.20 am | *Modelling human fertility and reproduction with stem cells*<br>Professor Amander Clark,<br>University of California, Los Angeles<br>25 MIN PRESENTATION + 10 MIN DISCUSSION |
| 11.55 am | **Description of breakout groups for Session 3 and explanation of roles (chairs, rapporteurs, participants)**<br>Professor Richard Harvey |
| 12.30 pm | Lunch |
| **Session 3 (Part 1): Breakout groups** | |
| 1.30 pm | Group 1 Platform science—Brisbane Room<br>Group 2 Clinical translation—Adelaide Room<br>Group 3 Public perspectives and expectations; Political and regulatory landscape—Darwin Room<br>Group 4 Current and future directions for stem cell science—Perth Room |
| 3.30 pm | Afternoon tea |
| 3.30 – 3.45 pm | **Closed session to discuss progress of group work (first ideas, overlaps, etc.)**<br>Chairs, rapporteurs and Academy secretariat |
| 4.00 pm | **Return to breakout groups**<br>Groups 2 and 3 joint session—Sydney Room<br>Groups 1 and 4 individual sessions |
| 5.30 pm | **End of Session 3** |
| 6.15 pm | Coaches depart for UNSW |
| 7.00 pm | **Pre-dinner tour** |
| 7.30 pm | **Dinner**<br>Scientia Centre<br>THIS FUNCTION PROUDLY SPONSORED BY<br>UNSW AUSTRALIA \| Science |
| 8.25 pm | **Guest speaker**<br>Nadia Rosenthal,<br>Australian Regenerative Medicine Institute |
| 10.00 pm | Coaches depart UNSW |
| 10.30 pm | Coaches arrive at hotel |

## *Day 3* Friday 24 July

| | |
|---|---|
| 8.30 am | **Session 3 (Part 2): Breakout groups Draft recommendations and presentations** |
| 10.00 am | Morning tea |
| 10.30 am | **Return to breakout groups to finalise recommendations and rapporteurs' presentations** |
| 12.30 pm | Lunch |
| **Session 4: Rapporteurs' presentations—Sydney Room** | |
| Chaired by Professor Edna Hardeman | |
| 1.30 pm | Group 1 **Platform science**<br>15 MIN TALK + 5 MIN QUESTIONS |
| 1.50 pm | Group 2 **Clinical translation**<br>15 MIN TALK + 5 MIN QUESTIONS |
| 2.10 pm | Group 3 **Public perspectives and expectations; Political and regulatory landscape**<br>15 MIN TALK + 5 MIN QUESTIONS |
| 2.30 pm | Group 4 **Current and future directions for stem cell science**<br>15 MIN TALK + 5 MIN QUESTIONS |
| 2.50 pm | **General open discussion** |
| 3.30 pm | **Wrap-up by session chair** |
| 3.45 pm | Afternoon tea |
| 4.00 pm | **End of the Think Tank for general participants** |
| 4.00–5.00 pm | **Closed session to prepare recommendations document**<br>Steering committee, chairs, rapporteurs and Academy secretariat |
| 4.30 pm | Coaches depart hotel for airport |

# THE PROCESS

## Day 1

### SESSION 1—OPENING SESSION: WELCOME AND SOCIAL EVENT

## Day 2

### SESSION 2—PRESENTATIONS SESSION

The plenary presentations are aimed at stimulating lateral thought in the discussions during the Think Tank, rather than providing comprehensive coverage of the theme or any of the four specialist topics.

### SESSION 3—BREAKOUT GROUPS (EXTENDS UNTIL MORNING OF DAY 3)

Each participant is assigned to one of four breakout groups and each group will be chaired by a member of the steering committee and will include relevant invited experts. Each group is made up of 15 researchers from across Australia and neighbouring countries with a mix of skills and experience, in order to stimulate lateral thinking and to challenge the participants to extend themselves and think dynamically. Two participants are preselected to act as the group's rapporteurs. The role of the rapporteurs is to collate the group's discussion and distil the discourse into a 15-minute presentation. The breakout groups are asked to examine and address their group's discussion questions but are also encouraged to move beyond these questions to other topics identified during the discourse.

## Day 3

### SESSION 4—RAPPORTEURS' PRESENTATIONS

The final half-day of the Think Tank will enable the group rapporteurs to synthesise the discussions and present a series of recommendations from each breakout group. There will be opportunities for questions and discussion after each presentation and during the general discussion.

At the end of the afternoon a closed session with the Steering Committee, experts, rapporteurs and Academy secretariat will summarise the outcomes of the meeting and plan the production of the recommendations document.

## Outputs

The rapporteurs, in consultation with their group, will be responsible for producing their group's contribution to the recommendation report. Each group report will extend to about 2–3 printed pages (1500 words) and consist of:

- a narrative summary of the key issues discussed
- recommendations for scientific research and science-based action that are needed in the short, medium and long terms.

The recommendations report from the Think Tank will be published and formally launched in late 2015. These recommendations will offer options for a 'way forward' and subsequently can be used to underpin policy development and research prioritisation. As has been the case for previous Think Tanks, it is expected that this report will be instrumental in influencing national policy development through government agencies.

# INTRODUCTION

While stem cell therapy has been part of medical practice for decades in the form of bone marrow transplantation and other treatments, recent advances have led to a revolution in biology and medicine that has not yet peaked. A key advance has been the development of human pluripotent stem cells. There is also a broadening awareness that all adult solid organs and soft tissues contain stem and progenitor cells that likely contribute to tissue homeostasis and repair after injury.

Stem cell science seeks to detail the diverse functions and regulation of stem cells in both developing and adult tissues. It aims to harness the knowledge learned for the creation of a new spectrum of therapies for genetic, injury, lifestyle and age-related diseases. Vigorous activity now occurs in all sectors of the basic and translational sciences surrounding stem cells. This activity interfaces with similar transformations occurring in genomics and epigenomics, human disease modelling and personalised medicine. Progress is welcomed and closely monitored by Australian patients and their families who see stem cell science and regenerative medicine as a means to alleviate their suffering.

It is imperative that the Australian science and biomedical research communities engage in stem cell research at the highest level to remain competitive internationally. It is unthinkable that Australia should fall short of contributing to the development of stem cell therapies and allied industries that will be part of precision and personalised medicine in

the future. We would also leave Australians with the perception that they are being denied potentially life-saving treatments.

In the current austere science funding environment, Australia must find the right balance for the future of stem cell science. How can we support and reward innovation in stem cell science? How can we facilitate the translation of discoveries into the clinic? How can we meet patient expectations while at the same time curb exploitative commercialisation of unproven stem cell therapies? How can the new generation of stem cell researchers with unique hybrid skill sets forge a career pathway?

The 2015 Theo Murphy High Flyers Think Tank will seek evidence-based policy solutions for these and other issues facing stem cell science in Australia.

# BREAKOUT GROUPS

**Colour coding**:

Group 1 Group 2 Group 3 Group 4

## Group 1
## Platform science

**Chair: Professor Martin Pera**

The fields of stem cell research and regenerative medicine are new, rapidly growing, and highly interdisciplinary. Stem cell science will have widespread ramifications across all areas of biomedical science, including functional genomics, developmental biology, disease modelling, drug development, and gene and cell therapies. Many now predict that regenerative medicine will become the fourth pillar of health care, alongside pharmaceuticals, biologics and medical devices. It is inevitable that future Australian biomedical science and health care will depend on maintaining and growing a rich and robust research effort in this domain. This in turn will depend on access to platform technologies that enable our researchers to stay on the frontiers of the field.

Technological advances, such as next-generation DNA sequencing or optogenetics, often reinvigorate existing research or open up entire new fields of investigation, and lead to the development of new diagnostic and therapeutic approaches. The scientific community must develop, identify and promote emerging technologies that will accelerate progress in basic and applied research. We must also pinpoint where current technology is lacking and new approaches are required.

Our understanding of the molecular regulation of stem cell behaviour has progressed enormously but there are still many gaps in our knowledge. Means to identify, characterise and manipulate stem and progenitor cells are constantly evolving, and progress towards constructing tissues and organs from cultured cells is accelerating.

Ancillary platforms such as cell culture technology and automation, targeted genetic manipulation, omics and bioinformatics, microfluidics, imaging, chemical biology, biomaterials and engineering are providing powerful new tools for stem cell science. In addition, pioneering early stage clinical trials are expected to pose new questions and challenges for basic research and reverse translation.

In this panel we will assess the current state-of-the-art of stem cell science nationally and internationally, and identify some of the areas where advances in technology are required to further both basic and translational research. We will also discuss barriers to dissemination of new technologies across disciplines and discuss strategies to surmount these hurdles. Finally, we will assess requirements for research infrastructure to support stem cell research.

### QUESTIONS TO GET YOU THINKING

1. What current scientific challenges in stem cell research cannot be tackled using current technologies?
2. What are the key technology gaps in cell therapy?
3. What infrastructure is required at a national level to accelerate the development of stem cell research and regenerative medicine?

### REFERENCES AND READING MATERIAL

Dimmeler S, Ding S, Rando TA and Trounson A. (2014) 'Translational strategies and challenges in regenerative medicine'. *Nature medicine* 20(8):814–821

Lancaster MA and Knoblich JA. (2014) 'Organogenesis in a dish: modelling development and disease using organoid technologies'. *Science* 345(6194): 1247125

Morris CA, Cahan P, Li H, Zhao AM, San Roman AK, Shivdasani RA, Collins JJ and Daley GQ. (2014) 'Dissecting engineered cell types and enhancing cell fate conversion via CellNet'. *Cell* 158(4):889–902

### GROUP 1 PARTICIPANTS (*RAPPORTEURS)

Associate Professor Maurice Curtis

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Dr Cathryn Haigh

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Dr Tim Hore

Dr Thierry Jarde

Dr Dhanisha Jhaveri

Dr Rajesh Katare

Dr Elizabeth Ng*

Dr Dmitry Ovchinnikov

Mr Gautam Wali

Dr Katharina Wystub-Lis

## *Group 2*
## *Clinical translation*

**Chair: Dr James Chong**

Whilst Australia has a rich history in the development of stem cell-based clinical therapies, a meaningful future role for Australia in the clinical translation of emerging therapies requires decisive action now. In North America and Europe many regulated clinical trials of experimental stem cell therapies have been initiated. In contrast, comparatively few trials are being initiated in Australia. In addition, regulatory 'loop holes' have opened the door for unregulated delivery of unproven stem cell treatments for commercial gain, involving many diseases in several organ systems.

Australia's relatively small population and biotechnology sector particularly disadvantage our participation in costly clinical translation in stem cell fields. It is vital that novel solutions are identified to address the hurdles impeding Australia's engagement in this potentially transformative medical field.

### QUESTIONS TO GET YOU THINKING

1. **Managing the 'valley of death'**: Translating stem cell therapies from the research 'bench' to the clinical bedside involves high cost and great effort both at the preclinical and the early-phase clinical trials stages. What novel strategies, designed to particularly suit the Australian stem cell environment, might give the most promising therapies the best chance of success?
2. **Cost of clinical trials**: Clinical trials—particularly late-stage efficacy studies—are extremely expensive. Stem cell therapies pose a very high risk investment and are more reliant on government funding rather than industry financing. Is the standard model of clinical trials viable for Australian clinical translation of stem cell therapies?
3. **Infrastructure for preclinical and clinical stem cell research**: In comparison to other regions around the world, Australia has not fostered the creation of specific infrastructure for successful preclinical and clinical translation of stem cell therapies. How can Australia best take advantage of currently available infrastructure and secure funds to build on this? What role should collaborative projects with other countries play within Australian programs?
4. **Careers**: With traditional grant funding at record lows, researchers in clinical translation are leaving research for more secure positions including those in provision of non-research industry services. With a specific focus on clinical translation of stem cell therapies, how can Australia best ensure that researchers are supported and career paths protected?
5. **Safety versus efficacy of therapies**: There are an increasing number of clinicians willing to charge patients for unproven treatments using purported 'stem cells'. Justifications given for such activities include:
   - the need for clinical exposure of novel and innovative therapies so that safety and efficacy can be tested
   - the high expense and long time-frame of conventional clinical trials
   - the unmet needs of patients, many of whom are desperate for treatment of their (often incurable) conditions.

   The current relatively unregulated environment means that there are few incentives for those working outside the proven framework of clinical trials to prove safety, disclose protocols, engage with peer-review or ethics committees and to report findings (including those that are adverse). This has led in some cases to a lack of transparency and even false advertising. How can Australia create a regulatory environment that ensures patient safety and enables the scientific community to learn from any clinical administration of stem cells whilst remaining sensitive to a patient's right to new and innovative therapies?

### REFERENCES AND READING MATERIAL

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Hey SP and Kimellman J. (2013) 'Ethics, error and initial trials of efficacy'. *Science Translational Medicine* 5(184):1–3

Main H, Munsie M and O'Connor MD. (2014) 'Managing the potential and pitfalls during clinical translation of emerging stem cell therapies'. *Clinical and Translational Medicine* 3:10

Tabar V and Studer L. (2014) 'Pluripotent stem cells in regenerative medicine: challenges and recent progress'. *Nature Reviews Genetics* 15(2):82–92

### *GROUP 2 PARTICIPANTS (*RAPPORTEURS)*

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Dr Leslie Caron
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Associate Professor David Curtis
Dr Mark Fear
Dr Julien Freitag
Dr Alexandra Harvey
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Dr Jennifer Hollands
Dr Joanna James
Dr Heather Main
Professor Iona Novak*
Mr Chih Wei Teng
Dr Tony White
Dr Yinghong Zhou

## *Group 3*
## *Public perspectives and expectations; Political and regulatory landscape*

**Chair: Professor Megan Munsie**

There is no doubt that stem cell science has captured public imagination—attracting both excitement about potential curative treatments for intractable conditions, and concern about the source of cells and technologies used by researchers. While community debate in Australia initially focused on whether it was 'ethical' to use human embryos in research, the last decade has seen mounting public frustration about the perceived lack of progress in delivering on the promise of stem cell science. This has resulted in the establishment of a thriving 'stem cell' industry—abroad and in Australia. The doctors and clinics involved are prepared to meet demand for treatment for a fee, despite the lack of reputable evidence on whether the interventions are effective or even safe.

If the Australian stem cell research sector is to remain viable, and importantly maintain community support and credibility, it is essential that researchers continue to engage with the broader community. Such engagement needs to accurately inform the public about research progress; identify, acknowledge and address ethical, regulatory and societal issues related to current and emerging technologies; and facilitate responsible clinical translation. Failure to do so will not only compromise the viability of the field, but in the case of unproven 'stem cell' interventions, place the health and wellbeing of patients at risk.

The task for Group 3 is to discuss public perspectives and expectation in stem cell science and determine whether current Australian regulations, guidelines and educational initiatives are adequate in light of current research practices and emerging technologies, as well as international standards. Members will be asked to develop recommendations to address any identified shortfalls.

### *QUESTIONS TO GET YOU THINKING*

1. **Do current regulations and guidelines strike the right balance between allowing research and addressing community concern about potential ethical issues?** For many years Australia has had permissive legislation in place to allow the use of human embryos (from donated excess in vitro fertilisation and those created via somatic cell nuclear transfer) in research, but are there additional research applications involving pluripotent stem cells that are likely to raise ethical concerns in the community? Consider the creation of human gametes; modification of human germline via CRISPR technology; research into mitochondrial disease, and the possibility of recapitulating early gastrulation in a dish. Do current legislation and guidelines address such issues? Are there any other emerging technologies or applications that may require refinement of current regulations or guidelines with respect to the use of pluripotent or indeed other types of stem cells? Relevance of current legislation and licensing requirements pertaining to the use of human embryos in research and prohibition of human cloning should also be considered.
2. **When should patients be able to access stem cell treatment?** Under what circumstances, if any, is it acceptable to apply experimental stem cell-based interventions in Australia? Should more be done to address medical travel abroad for putative stem cell treatments? Given this is happening, and will probably continue to happen, would it be informative to collate the experience of individual travellers and, if so, how could this be achieved?
3. **Who should have oversight of stem cell clinical translation in Australia?** Is the current exclusion of all autologous cellular therapies from TGA oversight acceptable? If not, what should be done? Should medical boards or the Australian Competition and Consumer Commission have a greater role in policy around sale of unproven treatments?
4. **Is there more that could be done to foster clinical translation of stem cell research in Australia?** What long-term strategy could be developed to enhance the number of clinical trials?
5. **Should more be done to engage the community about Australian stem cell science and if so, how?** In particular, how could patients be better informed of developments in the field and potential risks in pursuing unproven treatment (if this is acknowledged as an issue)? What role should scientists and regulators play in such engagement and who else should be involved? Should the stem cell research community do more to engage with media, politicians and policy makers, and if warranted how could this be achieved?

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Stem cells, cloning and related issues: https://www.nhmrc.gov.au/about/nhmrc-committees/embryo-research-licensing-committee/human-embryos-and-cloning/stem-cells-clon

Review of relevant chapters of the 'National Statement on Ethical Conduct in Human Research'. https://www.nhmrc.gov.au/health-ethics/national-statement-ethical-conduct-human-research/review-national-statement-chapter-3

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McLean AK, Stewart C and Kerridge I. (2015) 'Untested, unproven, and unethical: the promotion and provision of autologous stem cell therapies in Australia'. *Stem Cell Research & Therapy* 6:12

Therapeutic Goods Administration 2015 discussion paper on 'Regulation of autologous stem cell therapies'. https://www.tga.gov.au/consultation/consultation-regulation-autologous-stem-cell-therapies#documents; and the Australian Academy of Science's submission to the public consultation https://www.science.org.au/sites/default/files/user-content/tga-consultation-on-regulation-of-stem-cell-therapies.pdf. Other submissions: https://www.tga.gov.au/submissions-received-regulation-autologous-stem-cell-therapies.

Petersen A, Seear K and Munsie M. (2014) 'Therapeutic journeys: the hopeful travails of stem cell tourists'. *Sociology of Health & Illness* 36(5):670–685

Master Z, Robertson K, Frederick D, Rachul C and Caulfield T. (2014) 'Stem cell tourism and public education: the missing elements'. *Cell Stem Cell* 15(3):267–270


#### *Some examples of current educational material:*


ISSCR Closer Look at Stem Cells website: http://www.closerlookatstemcells.org/

NHMRC Stem cell treatments information: https://www.nhmrc.gov.au/guidelines-publications/rm001

Australian Stem Cell Handbook: http://www.stemcellfoundation.net.au/stem-cell-treatments-information/handbook

ASSCR Stem Cell Essentials: http://www.asscr.org/assets/ASSCR-SOC/Stem-Cells-Aust-WEB.pdf

EuroStemCell fact sheets: http://www.eurostemcell.org/stem-cell-factsheets

CIRM–For Patients website: https://www.cirm.ca.gov/patients


### *GROUP 3 PARTICIPANTS (*RAPPORTEURS)*

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Dr Lezanne Ooi

Dr Rebecca Pelekanos

Dr Nilay Thakar

Dr Jana Vukovic

## *Group 4*
## *Current and future directions for stem cell science*

**Chair: Professor Patrick Tam**

The conceptual paradigm of stem cell biology is founded on discoveries on the process of cell lineage differentiation in development, tissue homeostasis and cancer cell biology. In the embryo, populations of cells that exist transiently during development have been shown to be able to generate a wide range (and potentially all) of the cell types present in the adult organism. In the mature tissues that are constantly replenished during life, such as the gut epithelium, blood cells and the skin, or can launch repair and healing of damages, such as the liver and muscles, self-renewing cell populations with multi-lineage potency that act as the precursors of the tissue-specific cell types have been identified. Collectively these are known as adult (tissue) stem cells; the best studied among them are the haematopoietic stem cells, the intestinal stem cells and the skin stem cells. Putative stem cells have also been identified in embryonal carcinoma, where it has been demonstrated experimentally a single embryonic carcinoma cells can differentiate into the diverse types of tissues found in a teratocarcinoma. Research in stem cell biology has been escalated by the accomplishment of capturing stem cells from the developing embryo of the mouse and the human. That these cells can be maintained in vitro as eternally self-renewing pluripotent cell lines enables direct experimental manipulation and performance of multifaceted analysis of their stemness, lineage potency and functional attributes of differentiation. The seminal discovery of that terminally differentiated somatic cells can be reprogrammed to

become pluripotent stem cells that are similar to the embryonic stem cells has led to another quantum leap in the scope of research endeavour in stem cell biology.

Current research in stem cell biology can be broadly delineated into:

- identification and characterisation of the resident stem cells and their niche in embryos and adult tissues, and their role in development and tissue homoeostasis/ regeneration in model organisms
- study of human and animal development in patterned and three-dimensional in vitro differentiation models of pluripotent stem cells
- establishment, maintenance and transition of the stem cell state of embryonic, adult and induced pluripotent stem cells and the consequential functional attributes
- the differentiation potential of stem cells: lineage propensity and options
- characterisation of the roadmap and signposts of lineage differentiation of stem cells: cell biology and omics insights of in vitro directed differentiation and parallel proceedings in vivo
- mechanism, pathway and endpoints of cellular reprogramming to pluripotency and the acquisition of 'synthetic' cell fate through targeted reprogramming or trans-differentiation
- translation of the stem cell knowledge to application in medicine, drug research and biotechnology.

The first task of this discussion group is to take a snapshot of the advances that have been achieved in these research domains, the ongoing research endeavour and the likely outcome, and to reflect on how stem cell research in Australia is tracking and to take stock of what we have or would have accomplished in the context of the international effort and contribution.

### *QUESTIONS TO GET YOU THINKING*

1. Are there any doors that are yet to be opened in these research domains?
2. Could we forecast any new paradigm of stem cell research that may evolve from the current domains or through the multi-domain integration of knowledge and research expertise?
3. Are there any research domains that align well with our goals and priorities, and the national interest?
4. How are we going to position our research workforce to engage the future scientific endeavour in stem cell biology by, for example, building expertise, establishing infrastructure and enhancing the research environment?

### *REFERENCES AND READING MATERIAL*

Stem Cell Australia: 2014 Annual Report: http://www.stemcellsaustralia.edu.au/News---Events/News/Stem-Cells-Australia-2014-Annual-Report-now-available.aspx

Kobold S, Guhr A, Kurtz A and Loser P. (2015) 'Human embryonic and induced pluripotent stem cell research trends: Complementation and diversification of the field'. *Stem Cell Reports* 4(5):914–925

Hussein SMI et al. (2014) 'Genome-wide characterization of the route to pluripotency'. *Nature* 516: 198–206

Ben-Zvi D and Melton DA (2015) 'Modeling human nutrition using human embryonic stem cells'. *Cell* 161(1):12–17

### *GROUP 4 PARTICIPANTS (*RAPPORTEURS)*

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Dr Anthony Cook

Dr Kathryn Davidson*

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Ms Kathryn Futrega

Dr Daniel Heath

Dr Sara Howden

Dr Robyn Meech

Dr Peter Psaltis

Dr James Ryall

Dr Lincon Stamp

Associate Professor Ingrid Winkler

Dr Raymond Wong

# STEERING COMMITTEE

## *Professor Richard Harvey (co-chair)*

**Deputy Director**
**Victor Chang Cardiac Research Institute**

Richard Harvey received his PhD in 1982 from the University of Adelaide, training in molecular biology. He undertook postdoctoral studies in embryology at Harvard University with Doug Melton, and then moved to the Walter and Eliza Hall Institute in Melbourne, establishing an independent group. In 1998, he relocated to the Victor Chang Cardiac Research Institute, where he is currently Co-Deputy Director and Head of the Developmental and Stem Cell Biology Division. Richard holds the endowed Sir Peter Finley Professorship of Heart Research at the University of New South Wales. He is a Fellow of the Australian Academy of Science and a member of EMBO. His research has focused on the genetic basis of heart development and congenital heart disease mechanism, and more recently on the biology and origins of adult cardiac stem cells, and cardiac regeneration.

## *Professor Edna Hardeman (co-chair)*

**Chair (Research), Head of Anatomy and Cell Biology**
**School of Medical Sciences**
**UNSW Australia**

Edna Hardeman has made significant contributions in the fields of skeletal muscle development, gene regulation, and disease, through the use of transgenic and knock-out mice. Her laboratory has made mouse models for human muscle diseases, trialed therapies, discovered disease mechanisms, and is currently exploring novel methods of enhancing muscle stem cell transplantation. In addition, she is developing novel intracellular intravital imaging to visualise muscle function. She is a founder of the US FASEB conference 'Muscle Stem and Satellite Cells', and a member of the European Neuromuscular Centre International Consortium on Nemaline Myopathy. Edna is a founder of the NSW Cell and Developmental Biology Annual Symposium, currently in its 17th year. She is the Chair of the Australian Academy of Science National Committee for Biomedical Sciences, the Chair of the NHMRC Animal Welfare Committee, and a past President of the Australian and New Zealand Society for Cell and Developmental Biology.

## *Dr James Chong*

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**Westmead Millennium Institute**
**Westmead Hospital Department of Cardiology**

James Chong is passionate about translating findings from the field of Cardiac Regeneration into viable therapies. He is a practising cardiologist at Westmead Hospital with an interest in interventional cardiology, and leads a research group at the University of Sydney School of Medicine, Westmead Millennium Institute. His research has been published in high impact journals such as Nature and Cell Stem Cell. He recently was awarded the Heart Foundation NSW CVRN Ministerial award for Rising Stars in Cardiovascular Research. James trained in clinical cardiology at Westmead Hospital before completing his PhD at the Victor Chang Cardiac Research Institute under the mentorship of Professor Richard Harvey. This doctoral training focused on a previously unidentified population of cardiac stem cells. With the support of a Fulbright Fellowship and an NHMRC Biomedical Training Fellowship, he undertook postdoctoral training at the University of Washington with Professor Charles (Chuck) Murry. During this period he extended his interests in translational cardiac regeneration to include the use of pluripotent stem cells in small and large animal models of myocardial infarction.

## *Associate Professor Megan Munsie*

**Head—Education, Ethics, Law and Community Awarenesss**
**Stem Cells Australia, The University of Melbourne**

Megan Munsie is a scientist who combines her extensive technical expertise in stem cell research with an interest and understanding of the complex ethical, societal and regulatory issues associated with the field. She has been involved in stem cell research in Australia since 1996 and is currently based at The University of Melbourne where she heads the Education, Ethics, Law and Community Awareness Unit at the Australian Research Council funded Stem Cells Australia initiative. Over the last decade, she has contributed to the development of relevant policy in Australia and abroad, and has co-authored numerous educational resources for the public and health professionals. She is a

member of an international research team that is exploring community expectation in relation to stem cell science, and in particular 'stem cell tourism'. Megan is an advisor to several organisations including Chair of the International Society for Stem Cell Research's 'Closer Look at Stem Cells' task force and the Policy, Ethics and Translation Sub-Committee of the Australasian Society for Stem Cell Research. Megan has a Bachelor of Applied Science from Queensland University of Technology, a Masters in Reproductive Sciences and a PhD from Monash University. It was during her PhD in 2000—where she demonstrated that stem cells could be made from 'cloned' mouse embryos, the first proof-of-concept for therapeutic cloning—that she saw the need to be more engaged with regulators and the public. She has worked as an embryologist in IVF clinics and for an ASX-listed biotechnology company.

## *Professor Martin Pera*

**Director, Stem Cells Australia**
**The University of Melbourne**

Martin Pera is Professor of Stem Cell Sciences at the University of Melbourne, the Florey Neuroscience Institute, and the Walter and Eliza Hall Institute for Medical Research. He serves as Program Leader for Stem Cells Australia, the Australian Research Council Special Research Initiative in Stem Cell Sciences. His research interests include the cell biology of human pluripotent stem cells, early human development, and germ cell tumours. Martin was among a small number of researchers who pioneered the isolation and characterisation of pluripotent stem cells from human germ cell tumours of the testis, work that provided an important framework for the development of human embryonic stem cells. His laboratory at Monash University was the second in the world to isolate embryonic stem cells from the human blastocyst, and the first to describe their differentiation into somatic cells in vitro. He has provided extensive advice to state, national and international regulatory authorities on the scientific background to human embryonic stem cell research.

## *Professor John Rasko*

**Head of Gene and Stem Cell Therapy Program**
**Centenary Institute of Cancer Medicine and Cell Biology**

John Rasko is an Australian pioneer in the application of adult stem cells and genetic therapy. He directs the Department of Cell and Molecular Therapies at Royal Prince Alfred Hospital and heads the Gene and Stem Cell Therapy Program at the Centenary Institute, University of Sydney. John is a clinical hematologist, pathologist and scientist with a productive track record in gene and stem cell therapy, experimental haematology and molecular biology. In over 150 publications he has contributed to the understanding of stem cells and haemopoiesis, gene transfer technologies, oncogenesis, human aminoacidurias and non-coding RNAs. He serves on hospital, state and national bodies, including Chair of the Office of the Gene Technology Regulator—responsible for regulating all genetically-modified organisms in Australia—and Chair of the Advisory Committee on Biologicals, Therapeutic Goods Administration. Contributions to scientific organisations include co-founder (2000) and past-President (2003–05) of the Australasian Gene Therapy Society; Vice President, International Society for Cellular Therapy (2008–12) and founder (2009) ISCT-Australia. He is a founding Fellow of the Australian Academy of Health and Medical Sciences. He is the recipient of national (RCPA, RACP, ASBMB) and international awards in recognition of his commitment to excellence in medical research, including appointment as an Officer of the Order of Australia.

## *Professor Patrick Tam*

**Deputy Director**
**Children's Medical Research Institute**

Patrick Tam is the Deputy Director and Head of the Embryology Research Unit at the Children's Medical Research Institute. He is a Senior Principal Research Fellow of the NHMRC of Australia and Professor in the Discipline of Medicine, Sydney Medical School of the University of Sydney. Patrick's research focuses on the cellular and molecular mechanisms of body patterning during mouse development and the biology of stem cells. He pioneered the application of micromanipulation and embryo culture for analysing mouse embryos and examining the development of the head and embryonic gut. The embryological analysis undertaken by his team at CMRI has enabled the construction of a series of fate-maps revealing the organisation of the basic body plan of the early embryo. The in-depth knowledge of cell differentiation during early embryogenesis laid the foundation for directing the differentiation of stem cells into clinically useful cell types for therapy in regenerative medicine. Patrick is an editor of Development and member of the editorial board of Developmental Cell and Developmental Biology, Differentiation and Genesis. He is the Mok Hing-Yiu Distinguished Visiting Professor at the University of Hong Kong. In recognition of his research achievement, he was awarded the President's Medal of the Australia and New Zealand Society of Cell and Developmental Biology in 2007, and is a Fellow of the Institute of Biology (UK), the Australian Academy of Science, the Society of Biology (UK) and the Royal Society of London.

# INVITED EXPERTS

## Professor Amander Clark

**Vice Chair, Department of Molecular Cell and Developmental Biology**

**Director, Embryonic Stem Cell Derivation laboratory**

**Key member, Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research University of California**

Amander Clark is Professor and Vice Chair of the Department of Molecular Cell and Developmental Biology, Director of the Embryonic Stem Cell Derivation laboratory, and key member of the Eli and Edythe Broad Center of Regenerative and Stem Cell Research at the University of California Los Angeles. Her work is focused on the use of pluripotent stem cells to understand the cell and molecular basis of human reproduction and embryo development with a focus on germline epigenetic reprogramming. Her laboratory is currently funded by the National Institute of Child Health and Human Development, the Concern Foundation and the Alternatives Research and Development Foundation. Amander is a recipient of a Young Investigator Award from the Lance Armstrong Foundation, a Research and Career Development award from STOP Cancer, and a Young Investigator Award from the International Society for Stem Cell Research. She is a member of the Hinxton Group, an international consortium of scientists, ethicists and policy makers for the use of pluripotent stem cell derived gametes and the convergence of gene editing technologies and stem cell science.

## Professor Hans Clevers

**Professor of Molecular Genetics**
**Hubrecht Institute**

Hans Clevers' research has shaped our understanding of Wnt signalling in development and disease. The laboratory originally focused on T lymphocyte transcription factors (Tcf), having cloned Tcf1 in 1991. With the discovery that Tcfs are the final effectors of Wnt signalling, laboratory interests changed to the biology of Wnt signalling in intestinal self-renewal and cancer. His laboratory identified Lgr5 as a novel marker of adult stem cells and linked Wnt signalling with adult stem cell biology, currently his major focus of research. His distinguished career in research has been recognised through numerous prestigious awards and prizes, including the Spinoza Prize in 2001 and Breakthrough Prize in Life Sciences in 2013. Hans was Professor in Immunology at Utrecht University (1991–2002), director of the Hubrecht Institute (2002–12) and president of the Royal Netherlands Academy of Sciences (2012–14).

## Associate Professor Ian Kerridge

**Director, Centre for Values, Ethics and the Law in Medicine, University of Sydney**

Ian Kerridge is Director and Associate Professor in Bioethics at the Centre for Values, Ethics and the Law in Medicine at the University of Sydney, and Staff Haematologist/Bone Marrow Transplant physician at Royal North Shore Hospital, Sydney. He is the author of over 200 papers in peer-reviewed journals and five textbooks of ethics, most recently Ethics and Law for the Health Professions (Federation Press, 2013). Ian is Chair of the Australian Bone Marrow Donor Registry Research Committee and a member of the Australian Health Ethics Committee and NSW Health's Clinical Ethics Advisory Panel. His current research interests in ethics include the experience of illness, philosophy of medicine, public health, emerging and (re)emergent infections (including tuberculosis), conflict of interest, stem cells, end-of-life care, genomics, drug policy and organ donation.

## Associate Professor Jose Polo

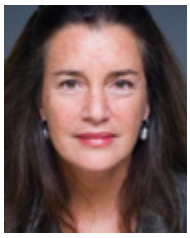
**Group Leader, Reprogramming and Epigenetics Laboratory**
**Monash University**

Jose Maria Polo was born in Buenos Aires, Argentina where he graduated from Buenos Aires University as a biochemist. In 2002, Jose began his graduate studies at Albert Einstein College of Medicine, New York under the supervision of Dr Ari Melnick where he worked on the transcriptional mechanism of the BCL6 repression complex in lymphomagenesis and B-cell maturation. In 2008 he obtained his PhD and moved to Boston to the laboratory of Dr Konrad Hochedlinger at the Harvard Stem Cell Institute to work on reprogramming of adult cells into induced pluripotent stem (iPS) cells. In particular, his work focused in the acquisition of immortality and the existence of epigenetic memory during reprogramming. In June 2011

as a Larkins Fellow, Jose established his independent research group at Monash University. In 2012, Jose was awarded a NHMRC Career Development Fellowship and in 2014 a Charles Viertel Senior Medical Research Fellowship to continue his work in the molecular mechanism governing the reprogramming process and stem cells epigenetics. As of 2013, Jose holds appointments to the departments of Anatomy and Developmental Biology and to the Australian Regenerative Medicine Institute.

## *Professor Nadia Rosenthal*

**Director**
**Australian Regenerative Medicine Institute**



Nadia Rosenthal is Founding Director of the Australian Regenerative Medicine Institute at Monash University and Scientific Head of EMBL Australia. She also holds a Chair in Cardiovascular Science at Imperial College London. She obtained her PhD from Harvard Medical School, where she later directed a biomedical research laboratory, serving for the New England Journal of Medicine as editor of the Molecular Medicine series. She established and headed the European Molecular Biology Laboratory (EMBL) campus in Rome for a decade. She is an EMBO member, a Fellow of the UK Academy of Medical Sciences, and was awarded the Ferrari-Soave Prize in Cell Biology and Doctors Honoris Causa from the Pierre and Marie Curie University in Paris and the University of Amsterdam. She is an NHMRC Australia Fellow. Nadia is a global leader in the use of targeted mutagenesis in animal models to investigate development, disease, and tissue repair. Her research focuses on the role of growth factors, stem cells and the immune system in the resolution of tissue injury for applications to regenerative medicine, and has led to significant advances in muscle ageing and heart disease. She participates on numerous advisory boards and committees including the European Research Council and Keystone Symposia. She is a Founding Editor of Disease Models and Mechanisms, and Editor-in-Chief of Differentiation and of a new Nature journal, Regenerative Medicine. Next year she will transit to The Jackson Laboratory in the US, where she will serve as Scientific Director.

## *Professor Paul Simmons*

**Head of Research and Development**
**Mesoblast Ltd**

Paul Simmons is currently Head of R&D at Mesoblast Ltd, a Melbourne-based regenerative medicine company founded in part on his work on prospective isolation of adult mesenchymal precursor cells (MPC). With a long and distinguished career in stem cell research, his contributions to the field over the past near 30 years were recognised by his election as President of the International Society of Stem Cell Research (ISSCR) in 2006. He received his PhD from the University of Manchester, UK in the laboratory of Mike Dexter, FRS and undertook postdoctoral training at the TFL, Vancouver, Canada and subsequently at the Fred Hutchinson Cancer Research Center, Seattle, USA. He was the inaugural R.L. Clifford Fellow in the Division of Haematology, Hanson Centre for Cancer Research, Adelaide, and subsequently Program Head in Stem Cell Biology at the Peter Mac. In 2002, he was part of the team of investigators who successfully competed for funding that led to the establishment of the Australian Stem Cell Centre (ASCC). Prior to joining Mesoblast in 2011, Paul was Director of the Centre for Stem Cell Research at the IMM, HTHSC, Houston, USA and held the C. Harold and Lorine G. Wallace Distinguished University Chair. He has served on editorial boards of multiple journals in the field of stem cell biology, is a member of F1000, and has received international recognition for his pioneering contributions to basic hematopoiesis research and of stem cells for the stromal system of the bone marrow.

# PARTICIPANTS

**Colour coding**:

Group 1 Group 2 Group 3 Group 4

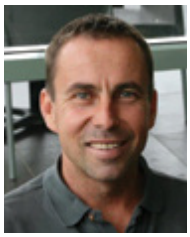
## *Dr Agnieszka Arthur* 4

**SA Pathology**
agnes.arthur@adelaide.edu.au

Agnieszka Arthur is a Mary Overton Research Fellow employed by The Royal Adelaide Hospital, located at the South Australian Health and Medical Research Institute (SAHMRI) working with Professor Stan Gronthos in the Mesenchymal Stem Cell Laboratory. She completed a Bachelor of Science at the University of Adelaide in 1999, with honours in the Department of Surgery (2000), investigating the involvement of the Eph/ephrin molecules in sciatic nerve injury and repair. She then worked as a research assistant for two years before commencing her PhD in 2003 through the University of Adelaide under the guidance of Professor Simon Koblar and Professor Stan Gronthos. During her PhD and thereafter she investigated the role of the Eph/ephrin molecules during dental pulp stem cell (DPSC) migration and differentiation. Secondly; she investigated the neural potential of DPSC, establishing novel (patented) techniques using an in ovo avian embryo model to investigate the neuroplasticity of DPSC and their therapeutic potential to treat the effects of stroke. With the support of the Mary Overton Research Fellowship and ADRF and NHMRC grants, Agnieszka has continued to investigate the function of the Eph receptor tyrosine kinase family during mesenchymal stem cell migration and endochondral differentiation and their importance during skeletal development and repair following trauma induced by tooth injury, fracture or osteoporosis. She has also investigated the supportive role of bone marrow derived MSC in lymphocyte function and haematopoietic support. This work has utilised both human samples, transgenic and conditional knockout mouse models.

## *Dr Guy Barry* 4

**Garvan Institute of Medical Research**
g.barry@garvan.org.au



Guy Barry is currently working in the laboratory of Professor John Mattick exploring non-coding RNA and elucidating the underlying mechanisms of human cognition and the implications for psychiatric disease such as schizophrenia and epilepsy in human neurons derived from induced pluripotent stem cells (iPSCs). IPSC-derived neurons provide for the first time the ability to manipulate and observe functional human neurons in culture and to then use techniques such as next generation sequencing to investigate primate- and human-specific genomic and transcriptomic changes. He collaborates closely with leading iPSC laboratories both locally and around the world to remain at the cutting edge of this exciting technology. Previously, he investigated brain development, especially the hippocampus, using mouse transgenic models. He has also had extensive experience in biotechnology while working at Neurocrine Biosciences Inc. located in San Diego, California. Guy's extensive molecular neuroscience and iPSC experience, coupled with interests in unravelling the mechanisms underlying human brain function and psychiatric disease, is well suited for this Think Tank. He will also be well placed in his senior role working with Professor Mattick to be involved in an Australia-wide effort in all areas of research under discussion during this event.

## *Dr Annemiek Beverdam* 4

**UNSW Australia**
A.Beverdam@unsw.edu.au

Annemiek Beverdam is a developmental geneticist with special interest in epidermal development and regeneration in the mouse, and in the genetic basis of human regenerative skin disease. She obtained her PhD in the laboratory of Dr Frits Meijlink at the Hubrecht Institute in The Netherlands in 2001. In 2002, she performed a short postdoc in the laboratory of Dr Giovanni Levi at the National Cancer Institute in Genoa, Italy. She then moved to Brisbane for a second postdoc in the laboratory of Professor Peter Koopman at the Institute of Molecular Biosciences, and she was senior postdoc in the laboratory of Professor Brian Key at The School of Biomedical Sciences at The University of Queensland. In 2013, she was recruited to the School of Medical Sciences, UNSW as an SPF03 strategic hire. She has published in leading journals including Journal of Investigative Dermatology, Development, and Human Molecular Genetics. The primary research focus of Annemiek's laboratory at UNSW is the genetic and molecular context in which Yes-associated protein (YAP) functions to control stem cell proliferation in normal epidermal homeostasis, how these processes are perturbed in regenerative skin diseases such as skin cancer, and to develop therapies to cure these disorders. Her research is funded by the NHMRC, and is part of a larger, long-term

and ambitious goal to identify key genes linking stem cell biology to organ size and growth.

## *Dr Alexis Bosman* 3

**Victor Chang Cardiac Research Institute**
a.bosman@victorchang.edu.au

Alexis Bosman is currently a Postdoctoral Research Fellow at the Victor Chang Cardiac Research Institute (VCCRI) in the laboratory of Professor Richard Harvey. Alexis received her training in cell and molecular biology at the University of Sydney (MSc) and the University of Geneva (PhD). Previous to her PhD studies, she held a Research Scientist position at Genea (previously Sydney IVF) working on the derivation of new human embryonic stem cell lines, both research and clinical-grade, from diseased and non-diseased embryos. During her doctoral studies, Alexis' research focused on the establishment of pluripotent cellular models of cardiac and hepatic disease using both human embryonic and induced pluripotent stem cells. Her current work at VCCRI focuses on modelling congenital heart disease using patient-derived induced pluripotent stem cells with the aim of uncovering the underlying cause/s of disease.

## *Professor Andrew Boyle* 2

**University of Newcastle, John Hunter Hospital**
andrew.boyle@newcastle.edu.au

Andrew Boyle is Professor of Cardiovascular Medicine at the University of Newcastle, Australia, and an Interventional Cardiologist at John Hunter Hospital. His laboratory studies the mechanism of heart failure after a myocardial infarction, and novel approaches to regenerate damaged heart muscle. Andrew's research has progressed from his PhD at the University of Melbourne, through his fellowship at Johns Hopkins University, and with his own laboratory at the University of California San Francisco from 2006 through 2014. His clinical research has involved stem cell therapy for myocardial infarction, from early investigator-initiated phase 1 pilot studies, through to participating in multi-centre clinical trials.

## *Dr Leslie Caron* 2

**Genea Biocells**
caron.leslie@gmail.com

Leslie Caron received a PhD from University of Nice-Sophia Antipolis, France where she worked on skeletal muscle differentiation of mouse embryonic stem cells (ESCs). Following her degree, she joined Professor Ken Chien's laboratory at Harvard Medical School, USA. Her postdoctoral studies focused on isolating cardiovascular progenitors from mouse and human ESCs. She showed that a triple-marked Isl1+/Nkx2.5+/Flk1+ multipotent progenitor gives rise to the three major lineages of the heart: cardiac myocytes, smooth muscle, and endothelial cells. This study provided new insights into the lineage diversification during mammalian heart development and represents a new strategy for cardiac tissue regeneration. In 2011, Leslie was awarded a fellowship from FSHD Global research foundation to derive skeletal muscle cells from Fascioscapulohumeral Muscular Dystrophy (FSHD) affected human ESC lines. Lack of a suitable cellular model for muscle diseases was stunting research into diseases like FSHD, with very little known and no progress in therapeutics. With 3 years of funding, she has developed a monolayer system capable of generating 70% mature and functional skeletal muscle myotubes in just 26 days. This protocol has been used to model FSHD phenotypes. It is now commercialised and is being used by top FSHD researchers in Europe and USA.

## *Dr Vashe Chandrakanthan* 4

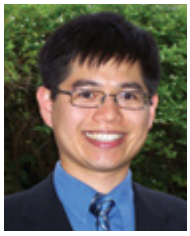
**UNSW Australia**
v.chandrakanthan@unsw.edu.au

Vashe Chandrakanthan received his PhD in 2008 from the University of Sydney, training in the field of early embryo development. He undertook postdoctoral studies in cardiac stem cell biology at the Victor Chang Cardiac Research Institute under the mentorship of Professor Richard P Harvey. In 2010, he moved to the Lowy Cancer Research Centre at the UNSW Australia. Vashe has established a research team with a specific focus on developmental biology of stem cells and their applicability towards tissue regeneration. The core focus of his research involves: (a) genetic fate mapping of mesenchymal stem-like cell (MS-LCs) origins and their contribution towards developmental hematopoiesis, (b) genetic fate mapping of embryonic mesenchymal stem cell-like cells (MSC-LCs) and vascular stem-like cells (VS-LCs) developmental origins and their contribution to coronary vascular development,

(c) applying Demethylation Cytokine induced (DCi) reprogramming technology and VS-LCs to regenerate damaged tissues, and (d) modifying the DCi reprogramming technology towards safety and efficacy for future human trials.

## *Associate Professor Fred Chen* 2

**Lions Eye Institute**
fredchen@lei.org.au



Fred Chen is a clinician-scientist at the UWA Centre for Ophthalmology and Visual Science and a Director for the Ophthalmic Research Institute of Australia. He is a consultant vitreoretinal surgeon and medical retina specialist at the Lions Eye Institute and Royal Perth Hospital. Fred received ophthalmology and retinal subspecialty training in Perth, Cheltenham and London. Following his return to Perth, after completing a PhD in retinal pigment epithelium transplantation in retinal diseases and fellowship training at Moorfields Eye Hospital, he established and directs the Ocular Tissue Engineering Laboratory at the Lions Eye Institute. He is also the co-director for the Imaging and Informatics Group and the director Clinical Research at the Lions Eye Institute. Fred has published over 70 research papers in the areas of retinal disease phenotyping, stem cell reprogramming and pigment epithelial cell transplantation for macular degeneration. He has received national and international research grants in multimodal retinal imaging and stem cell therapy. Currently, he is an NHMRC Early Career Fellow working on clinical application of microperimetry. He is a principal investigator in several clinical trials of novel therapies for dry and wet macular degeneration. Fred's special interests are in age-related macular degeneration, diabetic retinopathy, disease modelling in inherited retinal dystrophy, retinal imaging, clinical trials endpoints and novel surgical techniques.

## *Dr Anthony Cook* 4

**University of Tasmania**
anthony.cook@utas.edu.au

Throughout his career, Anthony Cook has actively sought research opportunities involving development and characterisation of human cell-based models to study the molecular and cellular biology of genotypic traits (pigmentation) and disease mechanisms (Parkinson's disease, glaucoma). He now leads a research team focused on development of a new human pluripotent stem cell-based model to study the degenerative, blinding eye disease glaucoma. His group has attracted substantial philanthropic funding for its work, and is supported by a national collaborator network of basic scientists and clinicians. Anthony also teaches cell biology, molecular biology, and genetics to undergraduate students.

## *Dr Kylie Crompton* 3

**Murdoch Childrens Research Institute**
kylie.crompton@mcri.edu.au

Kylie Crompton is research officer in the Developmental Disability and Rehabilitation Research group at Murdoch Childrens Research Institute, focused on regenerative medicine of the nervous system. Her background is based in applied science of tissue engineering for Parkinson's disease, and collaborative biomaterials work on regeneration of peripheral nerves and skin wounds. Currently, Kylie is preparing for an investigator-initiated clinical trial of cord blood stem cells as a potential therapy for cerebral palsy and has gained some experience with the ethical, governance and regulatory hurdles facing translation of stem cell research. She talks directly with families considering autologous stem cell treatments within Australia, or international stem cell tourism; and with clinicians under pressure to explain stem cells and the research timeline.

## *Associate Professor David Curtis* 2

**Monash University**
david.curtis@monash.edu

David Curtis completed his training as a Clinical Haematologist and Bone Marrow Transplant Physician through the University of Melbourne in 1994. He then completed his PhD at WEHI in 2000 in the laboratory of Professor Glenn Begley. After a three year post doctoral stint at the NIH, he returned to establish an independent research program using mouse models to understand the genetic and epigenetic regulation of hematopoietic stem cells with the goal of identifying new therapeutic approaches for blood cancers. He has also used mouse models to understand how hematopoietic stem cells can be used to improve heart function and repair following myocardial infarction. In 2011, David relocated from the Royal Melbourne Hospital to the Australian Centre for Blood Diseases, where he is Acting Head of Malignant Haematology Research. His work has been published in journals including Science, Cell Stem Cell, Nature Structural & Molecular Biology and Blood. In addition to his research program, he is an active clinical haematologist and bone marrow transplant physician at Alfred Health.

## *Associate Professor Maurice Curtis* 1

**University of Auckland**
m.curtis@auckland.ac.nz

Maurice Curtis grew up in rural New Zealand before moving to Auckland to study radiography. After completing radiography training he studied for a Masters of Science degree at the University of Auckland focusing on foetal stem cell transplantation for the treatment of neurodegenerative disorders such as Huntington's disease. Maurice then completed a PhD in anatomy and pharmacology under the guidance of Professor Richard Faull and investigated whether or not the human brain had the capacity to make new brain cells, which is a phenomenon thought not to exist in the brain. The findings from his PhD were ground breaking as he discovered that in human brains affected by Huntington's disease there is a massive increase in the amount of new brain cell production as the brain attempts to repair itself. These studies were published in a number of prominent journals and set the scene for much of the work that followed. In addition to receiving the University of Auckland's Best Thesis Award, Maurice was also awarded a Wrightson post-Doctoral Fellowship from the Neurological Foundation of New Zealand to work on stem cells in the brain at the University of Gothenburg in Sweden with Professor Peter Eriksson. During his work in Sweden, Maurice, together with colleagues in New Zealand, discovered a long distance migratory pathway through which stem cells in the brain migrate. This was a discovery of much interest and was reported around the world and published in the prestigious Science journal. This discovery also earned him the Royal Society of New Zealand's Hamilton prize in 2008. Maurice maintains close ties with the researchers in Sweden and other parts of Europe and is currently employed as an Associate Professor in the Department of Anatomy with Radiology at Auckland University where he is the deputy director of the human brain bank. He is a very active teacher and researcher. His research group consists of 15 scientists and together they research brain stem cells, stem cell migration, neurodegenerative diseases and neurorehabilitation at the recently-formed Centre for Brain research at the University of Auckland.

## *Dr Charbel Darido* 1

**Monash University**
charbel.darido@monash.edu

Charbel Darido completed his PhD studies on colon cancer at the University of Montpellier in France in 2008. For the last seven years he has worked with Professor Stephen Jane in Melbourne. In his first postdoc of three years at the University of Melbourne, he identified a critical tumor suppressor gene in skin squamous cell carcinoma (SCC). For the last four years, Dr Darido led the cancer program in the epidermal development laboratory at The Alfred Hospital, Monash University. His current research investigates SCC of multiple tissues including the skin, head and neck and oesophagus, and the cell of origin of these cancers, assumed to be a cancer stem cell (or stem cell-like). His ultimate goals are to translate molecular insights into novel therapeutic approaches. Charbel received the inaugural Clare Oliver Memorial Fellowship and multiple competitive funding including VCA, AICR and NHMRC grants. Recently he was appointed as a Group Leader at The Victorian Comprehensive Cancer Centre (VCCC) to start in 2016.

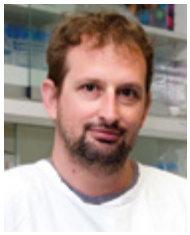
## *Dr Kathryn Davidson* 4

**Centre for Eye Research Australia and Australian Regenerative Medicine Institute**
kdavidson@unimelb.edu.au

Kathy Davidson has been involved in stem cell research in Australia and the US for over 14 years. She completed her PhD at Monash University in the laboratory of Professor Martin Pera in 2009, where she studied cell signalling in human pluripotent stem cells and neural progenitor cells. Kathy further pursued her interest in Wnt signal transduction in human stem cells as a postdoctoral fellow with Professor Randy Moon at the University of Washington. In 2013 she joined Associate Professor Alice Pebay's group at the Centre for Eye Research Australia where she established induced pluripotent stem cell-based disease models for macular degeneration as part of the Neuroregeneration program. She recently joined the Australian Regenerative Medicine Institute to undertake a new research direction aimed at harnessing knowledge of cell plasticity and pluripotency to develop cell-based therapeutics with Professor Andras Nagy, Associate Professor Jody Haigh, and Associate Professor Jose Polo.

## *Dr Nicolas Dzamko* 1

**Neuroscience Research Australia**
n.dzamko@neura.edu.au



Nic Dzamko is a research fellow at Neuroscience Research Australia and the Faculty of Medicine at the University of NSW. His focus is on understanding the genetic causes of Parkinson's disease and applying findings to develop biomarkers for earlier diagnosis and new therapeutics for treatments. Nic has a BSc(hons) degree from Flinders University and a PhD from the University of Melbourne. He has trained at the Garvan Institute of Medical research in Sydney and the MRC Protein Phosphorylation Unit in Dundee, Scotland. He currently holds funding from the Michael J Fox Foundation for projects involving generating IPS-derived models of Parkinson's disease.

## *Dr Michael Edel* 1

**University of Barcelona and University of Western Australia**
edel.michael@gmail.com

Michael Edel is a group leader and an accredited Associate Professor at the University of Barcelona, Faculty of Medicine with Spanish national project grant funding (equivalent to the Australian NHMRC grant system). He is an expert in cancer genetics and cell pluripotency. His current research is concerned with the role of the cell cycle in attaining a pluripotent state and the development of cancer. Michael leads a group dedicated to developing new methods to make genetically stable high quality clinical grade stem cells and pluripotent stem cells to study human lung, cardiac and neural disease in collaboration with clinicians both nationally and internationally. He is affiliated as a Senior Research Fellow at the University of Western Australia, School of Anatomy Physiology and Human Biology and The Harry Perkins Institute for Medical Research (CCTRM); Senior Research Fellow at the University of Sydney, Faculty of Medicine, Children's Westmead Hospital, Westmead, NSW; and Visiting Research Fellow at the Victor Chang Cardiac Research Institute, Sydney.

## *Dr David Elliott* 4

**Murdoch Childrens Research Institute**
david.elliott@mcri.edu.au

David Elliott completed his PhD in the laboratory of Professor Richard Harvey at The Walter and Eliza Hall Institute and The Victor Chang Cardiac Research Institute. The focus of his PhD was the characterisation of transactivation domains of the homeodomain protein Nkx2-5 and their role in heart development and disease. For his postdoctoral studies David studied *Drosophila* neurogenesis in the laboratory of Professor Andrea Brand at the Gurdon Institute, the University of Cambridge. He returned to Australia in 2007 to take up a postdoctoral position in the Embryonic Stem Cell Differentiation laboratory, jointly headed by Professor Andrew Elefanty and Professor Ed Stanley, at Monash University. During this time David developed a range of technologies and reagents to investigate human heart development using differentiating human pluripotent stem cells as a model system. In 2013 he started his laboratory at the Murdoch Childrens Research Institute. The focus of the laboratory is to investigate the genetic control of early human heart development and develop pluripotent stem cell based models of congenital heart disease.

## *Dr Mark Fear* 2

**University of Western Australia**
mark.fear@uwa.edu.au

Mark Fear graduated from Oxford University in 1996, subsequently working for two years at Northwestern University, Illinois, USA on haematopoiesis before completing his PhD (University of London) in the field of skin and skin cancers in 2003. Since 2006, Mark has worked with Professor Fiona Wood, establishing a basic science research program in the field of burn injury and scar formation. Mark currently works at the Burn Injury Research Unit at the University of Western Australia and is a board member of the Fiona Wood Foundation. He has a longstanding interest in cell therapies in the treatment of burn injury and scarring and more recently has also focused on cell programming in scarring and fibrotic disease.

## *Dr Julien Freitag* 2

**Melbourne Stem Cell Centre**
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Julien Freitag is a fellow of the Australasian College of Sports Physicians. His primary practice involves the active management of musculoskeletal conditions including osteoarthritis. He has published numerous articles, including those in peer-reviewed international journals, on the use of biological regenerative therapies. Julien obtained his medical degree at the University of Melbourne and completed a Bachelor of Medical Science at the Australian Institute of Sport, where he was involved in the development of a test for the detection of EPO abuse prior to the Sydney Olympics. He has worked as a medical professional at UCI World Cup cycling events and with Cycling Australia as the Doping Commissionaire at the Tour Down Under. Heavily involved in triathlons both as a doctor and as a competitor, Julien has represented Australia in triathlon at an international level and raced professionally on the European triathlon circuit. He is the clinical director of Melbourne Stem Cell Centre.

## *Ms Kathryn Futrega* 4

**Queensland University of Technology**
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Kathryn Futrega graduated with a BSc, Honours in biochemistry with a specialisation in biotechnology from the University of Waterloo, Canada. Through the UWaterloo Co-op Education program, she had the privilege to conduct research in molecular biology and biochemistry at UWaterloo and Cornell University, as well as review activity in the nanotechnology sector for Environment Canada. After completing her BSc, she relocated to the Queensland University of Technology at the Translational Research Institute to pursue a PhD in Biomedical Engineering. Kathryn's primary research interest is stem cell niche regulation and the development of in vitro tissue models that enable a more effective recapitulation of these complex microenvironments. Through her research she has made two specific contributions: (1) The development of a novel 3D tissue culture device termed the Microwell-mesh; and (2) the development of a 3D bone marrow niche model which is being optimised to enable bone marrow mimicry and as a delivery platform for cell-based transplantation. In general, her research looks to exploit engineering solutions to maximise the therapeutic potential of stem cells through ex vivo manipulation and to understand how such manipulations can prime cells to behave in vivo.

## *Associate Professor Jody Jonathan Haigh* 1

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Jody Haigh completed his undergraduate degree in life sciences (BSc.H.) and MSc degree in biochemistry at Queen's University in Kingston, Canada. This was followed by a PhD in biochemistry at the IMP/University of Vienna, Austria. He then spent four years as a postdoctoral fellow in the laboratory of Dr Andras Nagy at the Samuel Lunenfeld Research Institute at Mount Sinai hospital in Toronto, Canada. From 2004–13 Jody ran his own research group at the VIB and was an Assistant Professor at Ghent University in Belgium. In 2013 he moved to the Australian Centre for Blood Diseases (ACBD) at Monash University on a Larkin's Fellowship as an Associate Professor. Throughout his career he has developed and used novel mouse embryonic stem (ES) cell-based transgenic technologies to study genes involved in cardiovascular and hematopoietic development and disease-related processes. He has co-authored numerous publications on the role of VEGF signalling in organogenesis and disease processes including cancer. Recently his group has started to work on understanding the role that the epithelial to mesenchymal transition (EMT) transcriptional modulators of the ZEB and SNAI family play in blood development and leukaemia and leukemic stem cells. The group also has a long-standing interest in the molecular basis of cellular (de) differentiation and has recently published a novel Rosa26-iPS mouse model to assist researchers in understanding the molecular basis of cellular reprogramming and cellular memory. Jody has co-authored more than 50 research articles in journals such as Blood, Cell Reports, Nature Communications, Nature, Nature Medicine, and Nature Cell Biology. His publications have been cited more than 3200 times.

## *Dr Cathryn Haigh* 1

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Cathryn Haigh is a senior research officer in the Department of Medicine (Royal Melbourne Hospital) at the University of Melbourne. She began research in the area of neurodegeneration in 2002 as a PhD student at the University of Bath (UK) and relocated to Melbourne following her graduation in 2006. Since graduating she has published more than 20 articles as first or senior author, secured more than $450 000 in competitive research funding, presented her research at 10 international

meetings (including five oral presentations, three invited) and supervised eight research students. Her research focuses on the pathogenesis of prion disease; the role of the prion protein in toxic signalling during Alzheimer's pathology; neuroactive peptides for modulating neural stem cell growth and quiescence; and near infra-red, non-invasive imaging of neurodegenerative disease-induced brain cell death in live mice. Cathryn has a strong history of leading cross-disciplinary teams, with the resulting research published in some of the highest-ranked journals in her field, including Cell Research, Journal of Cell Science and Free Radical Biology and Medicine. Recently generated stem cell culture models have expanded her research directions into the control of neurogenesis and her research interests further extend into the area of therapeutic development for neural regeneration.

## *Dr Alexandra Harvey* 2

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Alex Harvey is a Postdoctoral Research Fellow in the School of Biosciences and Stem Cells Australia at the University of Melbourne. She received her PhD in obstetrics and gynaecology from the University of Adelaide investigating the role of oxygen in regulating preimplantation embryo development. Following her PhD, she worked for a commercial bovine embryo production facility in New Zealand, returning to Australia to undertake a commercial research project at the University of Adelaide for ICPBio NZ. In 2005 she moved to The University of New Orleans, USA to carry out postdoctoral work, shortly after which the laboratory was affected by Hurricane Katrina. Despite this interruption, Alex continued to focus on characterising mitochondrial properties of primate embryos and embryonic stem cells in response to culture. In 2007 the laboratory moved to Wayne State University, where her work continued to focus on the legacy of environmental (culture) effects on preimplantation embryo and embryonic stem cell development, characterisation and signalling. Returning to Australia in 2010, Alex worked with Vitrolife to develop a xeno-free embryonic stem cell medium. In 2011 she took up a postdoctoral position within Stem Cells Australia to investigate metabolic regulation of human embryonic stem cells and the impact of the extracellular and intracellular environment on human embryonic stem cell quality. During this time she has been able to revisit the role of oxygen in stem cell biology, establishing a model to identify incidences where metabolism is perturbed in stem cell populations.

## *Dr Daniel Heath* 4

**University of Melbourne**
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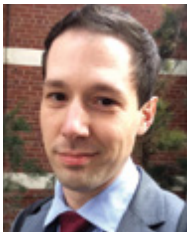

Daniel Heath is a lecturer in the Department of Chemical and Biomolecular Engineering at University of Melbourne. His research focuses on designing next generation biomaterials for controlling interaction between cells and the biological environment. He is particularly interested in designing materials that control the behaviour of stem cells (such as MSCs and EPCs). The end goals of Daniel's research are to build tissue engineering scaffolds that result in better healing and regeneration of tissue structure, improve the expansion of stem cells for use in stem cell therapies, and understand the heterogeneity of stem cell populations.

## *Dr Tracy Heng* 2

**Monash University**
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Tracy Heng obtained her PhD in immunology at Monash University in 2006. She moved to Boston for her postdoctoral training at Harvard Medical School, where she developed novel strategies for generating disease-specific regulatory T cells and established the Immunological Genome Project, a widely-used resource for expression profiling of immune cell populations. She returned to Monash University in 2009 and her current research aims to broaden the indications for stem cell therapies by overcoming their immunological barriers. Her group works on: 1) improving transplantation success in the aged by regenerating immunity with haematopoietic stem cells; and 2) understanding the immune interactions of mesenchymal stem/stromal cells and lymph node stromal cells to maximise their therapeutic potential. Her 30 publications since 2005 have accrued over 1430 citations and appeared in leading journals Nature Immunology, Immunity, and Science Translational Medicine. She is the recipient of an ARC Australian Postdoctoral (Industry) Fellowship and is Lead Chief Investigator of an ARC Linkage Project in partnership with Mesoblast.

## *Dr Jennifer Hollands* 2

**The Florey Institute of Neuroscience and Mental Health**
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Jennifer Hollands recently graduated from her PhD in which she studied human pluripotent stem cell (hPSC) differentiation, in particular, towards mesoderm derivatives, including blood cells, and primordial germ cells. She conducted this research under the supervision of Associate Professor Andrew Elefanty and Associate Professor Edouard Stanley through Monash University, Melbourne. Her PhD involved the generation of a number of genetically modified human embryonic stem cell (hESC) lines with the express purpose of assessing a human gene whose function was not known. The lack of knowledge about this human gene was largely owing to the absence of a rodent homologue, and thus the absence of a mammalian model system. Employing genetically modified hESCs she was able to show that this human gene is involved in germ cell development. This novel finding has expanded knowledge of the early stages of human development, with implications in human fertility and germ cell tumour formation. Jennifer is currently a postdoctoral researcher working with Dr Lachlan Thompson in the Neurogenesis and Neural Transplantation Laboratory, Florey Institute of Neuroscience and Mental Health, Melbourne. The key interests of this research laboratory are the in vitro derivation of neural derivatives from hPSCs and their transplantation into rodent models of human disease. One of her key interests is in the translation of medical research to the clinic, in particular in the exciting field of neuroscience. As such she aims to be a key driver in the development and translation of stem cell research in neural transplantation.

## *Dr Teresa Holm* 1

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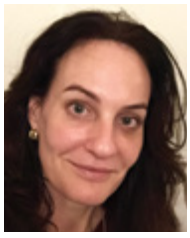

Teresa Holm's research is focused on using human induced pluripotent stem (iPS) cells and their application to human health. Pluripotent stem cells can form any cell type in the body and thus are a limitless source of tissue for transplantation. In addition, when iPS cells are derived from patients with genetic illnesses, they can be used to model the human disease in a dish. Teresa is interested in both disease modelling (with a current focus on cystinosis) and the 'directed differentiation' of iPS cells into cells of the kidney.

## *Dr Tim Hore* 1

**University of Otago**
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Tim Hore has recently established his research group within the Department of Anatomy at the University of Otago, following on from successful postdoctoral work at the Babraham Institute in Cambridge, UK. His research focuses on naive pluripotent stem cells and how their unique biological properties are shaped by epigenetic modification. In particular, Tim contributed to the discovery that naive pluripotent stem cells undergo genome-wide hypomethylation and that removal of DNA methylation by oxidation to hydroxymethylcytosine assists reprogramming of induced pluripotent stem cells (iPSCs) to the naive state. He is interested in the future applications of naive pluripotent stem cells and is actively researching the efficiency by which they can be created, and their ultimate safety for transgenic animal production and regenerative medicine.

## *Dr Sara Howden* 4

**Murdoch Childrens Research Institute**
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Sara Howden is a postdoctoral fellow at the Murdoch Childrens Research Institute. She received a BSc from the University of Melbourne and joined the Cell and Gene Therapy Lab at the MCRI to undertake an honours project. She stayed on to complete her PhD in the same laboratory before moving to Madison, Wisconsin for a postdoctoral position in the laboratory of Dr James Thomson. She has recently returned to complete the Australian component of her Overseas Biomedical research fellowship where she will continue to develop and apply the reprogramming and gene targeting methodologies she acquired during her time in the Thomson laboratory.

## *Dr Jo James* 2

**University of Auckland**
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Jo James is a lecturer and scientist in the Obstetrics and Gynaecology Department at the University of Auckland, New Zealand, where she leads a research group focused on the two types of stem cells from which the placenta is formed at the start of pregnancy: mesenchymal stem cells

and trophoblast stem cells. Her research uses these cells to help understand how stem cell function may contribute to normal and pathological placental development, with the long term goal of employing these stem cells as therapeutic agents or targets to treat placental pregnancy disorders. She is also interested in how the unique embryological nature of placental stem cells may be applied to other fields of regenerative medicine including cartilage repair and corneal regeneration. Jo is a council member of the Society for Reproductive Biology and a member of the Australian and New Zealand Placental Association Executive Committee. She teaches stage 3 and postgraduate reproductive biology courses.

## *Dr Thierry Jarde* 1

**Monash University**
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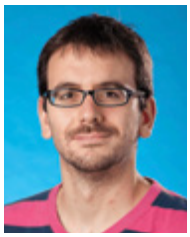

Thierry Jarde is an early career scientist who was awarded his PhD in Cellular and Molecular Biology in 2009. Following his postdoctoral studies at Cardiff University (UK), he was recruited in 2012 to the Department of Anatomy and Developmental Biology at Monash University. Thierry is interested in different aspects of adult stem cell biology. In particular, his research focuses on understanding the role of intestinal stem cells and their associated niche cells during normal homeostasis, age-related degeneration and tumourigenesis, with a specific emphasis on mechanisms that control stem cell behaviour. In addition, he is developing an in vitro living biobank that will allow normal and tumour stem cells to be screened for cancer drugs. This culture system is likely to represent the future of personalised therapies. Over the years, Thierry has had the chance to work within multiple research environments in France, UK and Australia.

## *Dr Dhanisha Jhaveri* 1

**Queensland Brain Institute, the University of Queensland**
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Dhanisha Jhaveri is a research fellow at the Queensland Brain Institute, where she discovered a distinct population of neural stem cells (NSCs) in the adult hippocampus that is activated by a subclass of clinical antidepressants via a novel pathway. More recently, she developed a new cell-sorting protocol that allows purification of endogenous hippocampal NSCs to homogeneity, providing critical evidence that distinct subpopulations of these cells exist in the adult brain. Her goal now is to elucidate the regulation and functional contribution of these subpopulations, which is essential to guide regenerative strategies for the treatment of learning- and mood-related disorders. The discovery of neural stem cells (NSCs) that drive neurogenesis in the adult mammalian brain has revolutionised our thinking about regenerative medicine. However, although research over the past two decades has made significant advances, several knowledge gaps remain before we can fully harness their potential in the treatment of neurological disorders. Amongst other factors, the quiescence and heterogeneity of NSCs have emerged as key principles in understanding their functional potential. Dhanisha's work investigating how environmental factors cause genetic and epigenetic changes that lead to subtype-selective activation of quiescent NSCs and their functional contribution in the adult brain therefore has immense translational value for ageing and regeneration. Given her passion for pursuing the promise and pitfalls of NSC research, she believes she can make a key contribution towards understanding the genomics of NSCs, as well as being part of the discussion regarding strategic directions for this research in Australia.

## *Dr Rajesh Katare* 1

**University of Otago**
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Rajesh Katare's research centres on identifying the mechanisms behind the development of cardiovascular diseases. In line with this, his research focuses on identifying the novel biomarkers for early diagnosis of the disease and developing novel therapeutic approaches to treat the disease. His group has identified stem cells as a promising candidate to treat the ischemic heart disease in patients who are resistant to the normal conventional therapies. In addition to publishing in a number of high impact publications Rajesh also collaborates with two biotechnology companies in the UK that develop stem cells for clinical therapy. One of the studies has now progressed into Phase I clinical trial in the UK. Rajesh is aiming to develop new approaches for stem cell delivery in to the heart that will improve the grafting, survival and differentiation of transplanted stem cells, aiding in regeneration of diseased hearts. Current ongoing stem cell research projects in his laboratory are supported by the funds from Heart Foundation NZ, JC Anderson Trust NZ and University of Otago.

## *Dr Henry Ko* 3

**University of Sydney**
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Henry Ko works in the fields of evidence-based practice, therapeutics industry governance and policy, and education at the NHMRC Clinical Trials Centre, the University of Sydney. He has worked in regenerative medicine since 2002, doing research and teaching at QUT, UNSW, the University of Sydney, Japan's National Institute of Advanced Industrial Science and Technology, the Hong Kong Polytechnic University, and a stem cell start-up company. In the policy and governance domain, he works with the Australian Government Department of Health's Medical Services Advisory Committee, Prosthesis Listing Advisory Committee, and the Australian New Zealand Clinical Trials Registry. He has also worked with the industry groups Medicines Australia and Medical Technology Association of Australia, and internationally on health technology assessment with Singaporean hospitals and the government. On the consumer and public side, Henry has a long-standing interest in public bioscience communication. He was a founding member of AusBiotech's student association in 2000 and was involved in promoting biotechnology to students and the public in various initiatives and forums in QLD and NSW until 2008. He has also talked about stem cells and biotechnology at National Science Week events. As a consumer advocate with the Consumers Health Forum of Australia since 2008, he has knowledge of health consumer policy issues. Henry was vice-president of the Asia Regional Young Investigator Chapter for the Tissue Engineering and Regenerative Medicine International Society from 2002 to 2008.

## *Dr Rebecca Lim* 3

**Hudson Institute of Medical Research**
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Rebecca Lim is a research group leader at The Ritchie Centre, Hudson Institute of Medical Research. She leads a team focused on uncovering the regenerative potential of amnion derived stem-like cells while concurrently pursuing clinical translation of her findings. In recent years, her team has uncovered key immunological events that are critical to the success of amnion cell mediated lung and liver repair in the settings of acute and chronic disease. Most recently she has shown that amnion cells trigger endogenous repair processes by activating the adult stem cell niche and recruiting distal progenitor sites to the area of injury. Research from Rebecca's laboratory is focused on understanding fundamental mechanisms of action in order to design informative clinical trials and best exploit the use of amnion cells in regenerative medicine. She is the chief investigator on a Phase I trial assessing the safety of intravenously delivered amnion cells in babies with established bronchopulmonary dysplasia.

## *Dr Tamra Lysaght* 3

**National University of Singapore**
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Tamra Lysaght is an Assistant Professor at the Centre for Biomedical Ethics at the National University of Singapore. Her research interests lie broadly in the ethical, sociopolitical and regulatory issues surrounding stem cell science and the clinical translation of regenerative medicines and genomics. She has expertise in empirical ethics and experience in using both qualitative and quantitative research methods. She has worked on policy issues with the Ethics Committee of the Human Genome Organisation, the Technical Working Group on Ethics at the World Health Organization and the Translational Clinical Research Programme of the Institute of Mental Health in Singapore, and the Human Health Division of the International Atomic Energy Agency. Tamra is currently working on the ethics and regulation of cell therapies and translational medicine in Asia and Australasia, and the ethics of One Health in Singapore.

## *Dr Casimir MacGregor* 3

**Monash University**
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Casimir MacGregor is a Research Fellow in the School of Social Science, Monash University. He is a medical anthropologist who specialises in the biopolitics of science and clinical medical anthropology. His PhD research examined the bioethical governance of human embryonic stem cell and cloning research in Australia. He is currently involved in an ARC-funded research project on stem cell tourism.

## *Dr Heather Main* 2

**Genea Biocells**
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Heather is a senior scientist with the Australian-owned embryonic stem cell company Genea Biocells. Her 12-year academic research career has seen her working in five countries, including PhD studies at Karolinska Institutet, Stockholm, Sweden. Heather's research has always been towards understanding regulation of cell fate decisions in embryonic stem cell neural differentiation and continues in her commercial research role. She has a passion for research innovation and translation and hopes to aid in developing the infrastructure and attitudes required for efficient translation of stem cell based therapeutics, in particular integrative pluripotent derived therapies.

## *Dr Dominique Martin* 3

**University of Melbourne**
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Dominique Martin is lecturer in health ethics within the Centre for Health Equity at the School of Population and Global Health at the University of Melbourne. A former medical practitioner with experience in emergency medicine, Dominique is a bioethicist whose research focuses on ethical issues relating to procurement, distribution and use of human biological materials and medical products of human origin. She is particularly interested in transnational markets for human organs, cells and tissue; international travel for medical care; the ethics of ageing; and issues in professional ethics. Dominique's current research projects include an investigation of issues in medical ethics related to the use of unproven stem cell interventions, and studies of public attitudes towards organ donation and transplantation in Sri Lanka and among different nationality groups resident in Qatar. Dominique has more than 25 publications in peer-reviewed journals and books, and regularly presents at international meetings in bioethics and transplantation. She teaches postgraduate courses in public health ethics and the ethics of ageing. Currently, Dominique is co-chair of the Ethics Committee of The Transplantation Society, and a member of the Executive Board of the Declaration of Istanbul Custodian Group. She has previously worked as a consultant ethicist to the World Health Organization and to the Qatar Organ Donation Centre.

## *Dr Robyn Meech* 4

**Flinders University**
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Robyn Meech is a stem cell biologist and pharmacologist with expertise in gene regulation, cell signaling, and stem cell function in contexts of disease, development, and ageing. She trained at Flinders University in South Australia and at The Scripps Research Institute in California. She currently directs a program on muscle stem cell biology with particular focus on the Wnt and Notch signalling pathways in the control of self-renewal, proliferation and differentiation. Robyn has received three NIH grants in support of her work and currently holds an ARC Future Fellowship to study epigenetic regulation of muscle stem cells using transgenic mice and next-gen sequencing methods. Her long-term focus is on how critical stem cell signalling and transcriptional pathways may be pharmacologically manipulated to aid tissue regeneration. Bridging her expertise in stem cells and pharmacology, she is also interested in drug metabolism in stem cells, and she currently holds an NHMRC project grant on drug metabolising enzymes that provide crosstalk to cell signalling and transcription by also metabolising endogenous signalling molecules and transcription factor ligands.

## *Dr Elizabeth Ng* 1

**Murdoch Childrens Research Institute**
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Elizabeth Ng's research is focused on the biology and manipulation of human pluripotent (hESC and hiPSC) stem cells. Her interests specifically lie in the regulation of human pluripotent stem cell differentiation to mesendodermal precursors (corresponding to the primitive streak in the mammalian embryo) and then to mesodermal lineages, as exemplified by blood cell and endothelium. To facilitate the optimisation of differentiation protocols for human definitive blood cell lineages, she has utilised genetically modified human ES cell lines generated in her laboratory, into which reporter genes have been inserted by homologous recombination in gene loci that are expressed during mesoderm, blood and endothelial development. A major goal of Elizabeth's work is to realise some of the scientific and therapeutic potential that hESCs and hiPSCs promise. These include unique opportunities for the study of early stages of human development, the generation of in vitro models for human

diseases, testing of pharmaceuticals and other therapeutic products and the production of transplantable cells for tissue repair and regeneration. A necessary requirement for the progression of both these research and therapeutic agenda is the ability to reproducibly and robustly direct the differentiation of human PSCs towards designated lineages. She has devised a reproducible differentiation protocol for human PSCs cells ('spin EBs'), complemented with an animal product free, recombinant human protein containing differentiation medium (APEL). Her APEL medium has been licensed and is produced by STEMCELL Technologies, a Canadian biotech company.

## *Dr Jonathan Niclis* 3

**The Florey Institute of Neuroscience and Mental Health**
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Jonathan Niclis began his research career at Monash Immunology and Stem Cell Laboratories with his PhD under the supervision of Alan Trounson. This involved the investigation of human embryonic stem cells (hESCs) that carried mutations for Huntington's disease. Upon completion of his PhD which established a novel neural differentiation protocol and insights into diseased hESCs, Jonathan joined the Florey Institute in 2012. At the Florey, Jonathan established a stem cell core within the Parish Laboratory and is driving several projects revolving around the derivation of specific neuronal populations from human pluripotent stem cells. In most cases these human neurons are utilised in a cell transplantation context, to replace cells lost in neurodegenerative disease and trauma events, such as Parkinson's and stroke. Crucially, this approach targets the cause of these disorders, the loss of neurons, and preliminary results demonstrate robust and reproducible outcomes of attenuated pathologies.

## *Professor Iona Novak* 2

**University of Sydney**
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Iona Novak is the Head of Research of the Cerebral Palsy Alliance Research Institute, School of Medicine, University of Notre Dame Australia. She is a Fulbright Scholar and winner of the University of Sydney Award for Professional Achievement. In 2005 she co-founded the Cerebral Palsy Alliance Research Institute with the Chief Executive Officer to develop and disseminate research leading to the prevention, cure and reduction of adverse effects for those living with cerebral palsy. Driven by an internal belief that health care truly has the potential to change lives, Iona has pursued projects and roles with the greatest possible current and future impact on children and families, for example leadership of the Australian Cerebral Palsy Register. Iona has a background in occupational therapy, with a particular interest in neuroplasticity. Her PhD work was in the area of home program intervention for children with cerebral palsy. In her current work, she has continued her research interest in evidence-based practice, knowledge translation, goal-directed training, botulinum toxin and population studies for cerebral palsy. In the last 10 years she has given over 100 international and national keynotes and invited lectures and been award over $16 million in research grants funding.

## *Dr Carmel O'Brien* 3

**CSIRO**
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Carmel O'Brien's career encompasses more than 15 years specialising in the study of human stem cell biology and extensive experience managing collaborative stem cell research programs in industry and medical research sectors (Monash IVF; Melbourne IVF; Stem Cell Sciences; CSIRO). Following graduation in clinical biochemistry, she pursued postgraduate studies in developmental biology, was awarded clinical embryology accreditation, a Masters degree (Monash Institute for Medical Research, 1993), and later a PhD degree (Monash University, 2002) for research recognised by a Keith Dixon Award in Developmental Biology (ANZSCDBI), Gordon Research Conference (USA) and MIMR postgraduate awards. Postdoctorally, Dr O'Brien has been directly involved in the human embryonic stem cell revolution in Australia, liaising with the NHMRC to attain a license for consented use of excess IVF embryos and pioneering derivation of Australia's first human embryonic stem cell lines for research use. Carmel has extensive research experience with Australia's first stem cell biotech SCS P/L (nine years) and since 2009 with CSIRO, working to understand stem cell 'decisions' in vitro, and develop technologies to aid quality control of stem cell applications. Her current research focus (funded by the governments of Australian and California) is to validate and exploit the properties of human embryonic (hES) and induced pluripotent (hiPS) stem cells to develop innovative research tools and solutions for longer-term clinically relevant applications (multiple sclerosis, breast cancer). Dr O'Brien is an active member of the stem cell research communities in Australia and globally, and is committed to secondary, tertiary and public education and debate in this field.

## *Dr Michael O'Connor* 3

**Australasian Society for Stem Cell Research**
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Michael O'Connor is a senior lecturer in the School of Medicine at the University of Western Sydney. His group uses human pluripotent stem cells to model the molecular development and diseases processes of the retina and lens. Michael is also the current President of the Australasian Society for Stem Cell Research, in which position he is developing integrated public engagement events to generate community discussion on stem cell research, clinical translation, and stem cell tourism. These events include the travelling Stem Cell Stories poster exhibition, internal and external building projection art, and a stem cell information brochure and mobile app. The Stem Cell Stories exhibition has been shown across Australia including in Canberra, Adelaide and Perth. It has been viewed by more than 500 000 people in person, with touring plans through to 2018. More than 280 000 people have also been engaged through TV and print media including the December 2014 issue of Cosmos magazine.

## *Dr Lezanne Ooi* 3

**University of Wollongong**
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Lezanne Ooi joined the University of Wollongong in 2012 and is a Senior Lecturer in Biological Sciences. Following a research post in neuroscience at GlaxoSmithKline (UK), she carried out her PhD at the University of Leeds (UK), studying gene regulation in neurodegenerative disease. During her postdoc she used advanced imaging, electrophysiology and behavioural experiments to understand the control of neuronal excitability and function. Lezanne's research interests include the use of induced pluripotent stem cells in neurodegenerative disease modelling and drug discovery and the regulation of neuronal function by inflammation and oxidative stress. Since 2012 she has published 18 articles and received $1.5 million in research funding. She served on the American Association of Anatomists Advisory Committee (2009–12) and is an editorial board member for Scientific Reports.

## *Dr Dmitry A. Ovchinnikov* 1

**University of Queensland**
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Dmitry Ovchinnikov is currently a Research Fellow in the Stem Cell Engineering Group at the Australian Institute for Bioengineering and Nanotechnology at the University of Queensland. His main interest is in the genetic manipulation of human pluripotent stem cells for investigations into the mechanisms of neurodegenerative diseases such as Down's syndrome and Alzheimer's disease. In particular, he is using both gene knockdown and overexpression approaches to dissect the roles of different signalling pathways and specific genes in the aetiologies of these conditions. Generally, Dmitry's research interests include the generation of transgenic ESC and iPSC research tools for bioengineering applications, re-programming somatic cells to pluripotency (iPSC generation), and approaches to control the undesirable de-differentiation and proliferation of stem cell-derived grafts. More recently, he has also developed an interest in the epigenetics of pluripotent cells, their derivatives and cancer cells. Previously, he led the Transgenic subgroup in the laboratory of Professor David Hume at UQ's Institute for Molecular Biosciences, where he generated a number of transgenic mouse lines, some of which are widely used for investigations into macrophage function and other in vivo studies. Dmitry received his Diploma in Biochemistry (Masters equivalent) from the M.V. Lomonosov's Moscow State University in 1993. He completed his PhD studies in the laboratory of Professor Richard R. Behringer at the University of Texas MD Anderson Cancer Center, where he was involved in the development of transgenic resources and performed some of the first conditional knockout studies in mice utilising the Cre/loxP system.

## *Dr Rebecca Pelekanos* 3

**The University of Queensland**
r.pelekanos@uq.edu.au

Rebecca Pelekanos was awarded her first large research grant as CIA ($145,000) in 2014, to investigate stem cell therapies in a rodent model of cerebral palsy. In previous appointments, she was involved in the clinical production of placenta-derived MSC for clinical trial and the comparison of MSC from different murine and human organs. In 2010, Rebecca was awarded a prestigious

Australian NHMRC Postdoctoral Training Fellowship that is allowing her to independently develop her research interests, and she has attained more than $500,000 funding in the form of grants, fellowships and travel awards. She was awarded a Dean's commendation for her PhD thesis in 2008 on growth hormone receptor activation mechanisms (top 10% of PhD graduates at the University of Queensland) and has presented her research at numerous local, national and international conferences. She is an author on 15 peer-reviewed publications in journals including Science, Nature Structural and Molecular Biology, and Stem Cells Translational Medicine. Her research is diverse and she is starting to create her own niche: molecular endocrinology in the fields of stem cells, paediatrics and pregnancy. Rebecca's focus has recently shifted to combining these areas of expertise to find novel stem cell and hormone treatments for cerebral palsy.

## *Dr Peter Psaltis* 4

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Peter Psaltis is a Clinical Academic and Interventional Cardiologist at the University of Adelaide and Royal Adelaide Hospital, and Co-Director of the Vascular Research Centre, South Australian Health and Medical Research Institute. After training in cardiology, he undertook his doctoral research in Adelaide from 2006 to 2009 investigating mesenchymal stem cell therapy for heart failure. His large animal studies were the first to validate the use of the NOGA® electromechanical mapping system to deliver stem cells transendocardially in nonischemic cardiomyopathy. From 2010 to 2012, he worked in the laboratory of Robert Simari, Mayo Clinic, Rochester, Minnesota as an NHMRC postdoctoral research fellow. There he discovered and characterised the presence of resident macrophage progenitor cells in the adventitia of postnatal arteries. He then undertook training in interventional cardiology at Monash Medical Centre, Melbourne before returning to Adelaide in 2015. Peter's research focuses on the origins and regulation of macrophage subtypes in the vasculature, the use of nanotechnology for macrophage-based imaging and intervention, and the therapeutic application of adult stem cells in atherosclerosis and cardiomyopathy. He has published over 55 full-text manuscripts, and is Associate Editor for Heart, Lung and Circulation and on the editorial board of the Journal of Geriatric Cardiology. His research discoveries have received numerous awards, including the ATVB Council's Early Career Investigator Award at the American Heart Association Meeting in 2011. He holds research grants and fellowships from the NHMRC and National Heart Foundation of Australia.

## *Dr James Ryall* 4

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James Ryall received his PhD in 2006 under the supervision of Professor Gordon Lynch of the Department of Physiology, the University of Melbourne. In 2008 James was awarded a CJ Martin overseas biomedical research fellowship (NHMRC) and joined the Laboratory of Muscle Stem Cells and Gene Regulation, run by Dr Vittorio Sartorelli, National Institutes of Health, US. James's work in the US identified a novel process of metabolic reprogramming in skeletal muscle stem cells, and linked this process to the regulation of stem cell fate decisions. This work was recently published in the prestigious journal Cell Stem Cell. At the end of 2012 he returned to Australia, and the Department of Physiology where he currently leads his own research group within the Basic and Clinical Myology Laboratory of Professor Gordon Lynch, focusing on the metabolic regulation of adult skeletal muscle stem cells and how this can be applied to regenerative medicine.

## *Dr Lincon Stamp* 4

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Lincon Stamp's research has focused on the derivation, isolation, characterisation and transplantation of stem cells from a variety of sources. He did his doctoral studies under the supervision of Professor Martin Pera. The research involved investigation of the early differentiation of human ES cells toward the endodermal lineage, with a focus on hepatopancreatic development. This work led to two co-first author publications in Stem Cells journal, as well as the granting of an international patent on which Lincon is a co-inventor. He then joined the laboratory of Professor Heather Young as a postdoctoral researcher at the University of Melbourne Department of Anatomy and Neuroscience, where he is working to develop a stem cell therapy to treat enteric neuropathies. Here the focus is on the development of neural crest and the enteric nervous system, and development of a small animal model of neural crest stem cell transplantation to treat enteric neuropathies. In particular, the research has aimed to develop a stem cell therapy to treat the paediatric enteric neuropathy, Hirschsprung disease. This work has led to a co-first author publication in March 2013 in the high-ranking journal, Journal of Clinical Investigation. In this world first study, Lincon and colleagues showed for the first time that

neural stem cells transplanted into the postnatal colon of mice can generate functional neurons of the appropriate neurochemical and electrophysiological phenotype.

## *Mr Chih Wei Teng* 2

**Monash University**
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Chih Wei Teng graduated from the National University of Singapore with a business degree focusing on operations management and e-business. He subsequently worked for IBM for seven years where he handled portfolios of energy and utilities, manufacturing, and healthcare and life sciences industries. Chih Wei completed his MBA from Monash University while working for a consulting company specialising in operations research. He is currently pursuing his PhD studies focusing on multi-disciplinary collaboration in the translation and commercialisation of regenerative medicine. Other research interests include innovation management, in particular the applicability of an open innovation framework in regenerative medicine; business models in autologous stem cell therapies; and impacts of formal and informal regulation in the provision of stem cell therapies. He has conference papers accepted and presented in a number of international conferences including EUROMA (European Operations Management Association), International Working Seminar on Production Economics in Innsbruck as well as scientific conferences in RMSC (5th Annual World Congress of Regenerative Medicine and Stem Cells) in 2012 and ISCT (International Society for Cellular Therapy) in 2014. In addition, he has written a diploma thesis on the changing nature and potential business models for cord blood banking as well as publishing three papers in A*- and A-ranked management journals.

## *Dr Nilay Thakar* 3

**Stem Cells Limited**
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Nilay Thakar completed his PhD in the field of stem cell biology under the supervision of Associate Professor Ernst Wolvetang at the University of Queensland in 2014. During his PhD he investigated the role of NFKB signalling in human embryonic and induced pluripotent stem cells, authored seven publications, served as a panellist on the ISSCR Junior Investigator Committee, won 14 scholarships and awards and was selected from more than 11 000 graduates as a University of Queensland Class of 2013 Future Leader for displaying great depth and diversity in leadership (Top 2% Graduand). Between 2010 and 2014 Nilay worked as a Commercialisation Analyst intern at UniQuest Pty Ltd helping negotiate with Janssen-Cilag Pty Ltd for a rheumatoid arthritis therapy, built an online platform for university graduates to secure internships with Fortune 500 companies, started a web-design consultancy serving Australian biotech spin-offs and, in 2013, was chosen by Queensland Premier Campbell Newman as the Premier's Open Data Award Finalist for conceiving an app for blind and deaf Australians to use public transport independently. He is also a co-founder of Defend—a program he is currently working on.

## *Dr Jana Vukovic* 3

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Jana Vukovic graduated from The University of Western Australia in 2004 with a Bachelor of Science (Honours), majoring in both neuroscience and genetics. She was awarded her PhD from the same institution in 2008. She then relocated to the University of Queensland to join Professor Perry Bartlett's laboratory at the Queensland Brain Institute as a Postdoctoral Research Fellow. She was awarded a Queensland Government Smart Futures Fellowship in 2010. In 2015, Jana established her independent laboratory with the School of Biomedical Sciences and the Queensland Brain Institute. Her laboratory investigates how microglia, the brain's resident immune cells, influence the process of learning and memory in ageing and disease. She is a recipient of an Australian Research Council (ARC) Discovery Early Career Research Award (2015–17).

## *Mr Gautam Wali* 1

**Griffith University**
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Gautam Wali is interested in using adult stem cells to understand the disease mechanism and screen drugs for Hereditary Spastic Paraplegia (HSP). Like many other neurological disorders, in HSP a particular population of brain cells—the cortical neurons—degenerate, leading to spastic paraplegia. To understand the fascinating biological mechanism of selective neuronal degeneration in HSP and its mediating pathways, he studied patient stem cells from the olfactory tissue. Olfactory stem cells have previously been used as cellular models to study diseases such as Parkinson's disease, Alzheimers disease and other motor

neuron diseases. As an outcome of this research, Gautam and colleagues have proposed a mechanism involving a cascade of events leading to cellular degeneration in HSP. HSP mutations in patient cells lead to reduced levels of stable microtubules (railway tracks of cells), which slows organelle (cargo) trafficking and leads to increased oxidative stress, which can cause cellular degeneration. They also showed that specific microtubule-binding drugs could revert this downstream effect. To validate the findings, Gautam and colleagues are working on generating induced pluripotent stem cells derived cortical neurons. The bigger aim of this research is to suggest potential drugs for HSP for clinical trials. He has also contributed to disease modelling of Ataxia telangiectasia. Gautam Wali is a PhD student and a Research fellow.

## *Dr Tony White* 2

**Monash University**
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Tony White is a physician-scientist in the cardiology field and an interventional cardiologist at MonashHeart, a busy public hospital cardiology unit in South East Melbourne. He has a great interest in stem cell therapy and attempts at regeneration of cardiac tissue. He sees particular promise in the potential of pluripotent stem cells, reprogramming technologies (iPS and in situ transdifferentiation) and DNA editing technologies such as CRISPR/Cas9. Tony did a three-year postdoc (2007–09) in Eduardo Marban's laboratory, performing preclinical testing of intra-myocardial injection of cardiosphere cells in a model of post-myocardial infarction heart failure (Cedars-Sinai Medical Center, Los Angeles). He is strongly influenced by mentors Professor Richard Larkins and Professor Graham Brown to strive to be an individual who can straddle the worlds of clinical medicine and basic science.

## *Associate Professor Ingrid Winkler* 4

**University of Queensland, Mater Research Institute**
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Ingrid Winkler is a Senior Research Fellow and head of the Stem Cells and Cancer group at Mater Research Institute, University of Queensland. Her research focuses on understanding how micro-environments protect and instruct stem cells. She has recently navigated the path from patenting basic scientific discoveries, to establishing relationships with various US biotech industry partners and commercialisation, to preclinical contract work and advisory panels and finally phase I/II clinical trials (in progress). Ingrid's innovative research has already led to several discoveries, including novel strategies to protect normal haematopoietic stem cells from chemotherapy or radiation damage, helping to alleviate life-threatening side-effects of cancer therapies, as well a novel strategy to chemo-sensitise leukaemia by therapeutic manipulation of specific niche components. In the last five years she has published three seminal papers on three novel aspects of stem cell niche biology. These include research on hypoxia and blood perfusion, research into macrophages, and a landmark study in Nature Medicine 2012, which described the role of a key component of the bone marrow vascular niche (E-selectin) in regulating haematopoietic stem cell self-renewal and chemo-sensitivity. In 2013, the latter research was recognised to be among 10 of the best research projects in Australia by NHMRC.

## *Dr Raymond Ching-Bong Wong* 4

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Raymond Wong is a stem cell biologist specialising in human pluripotent stem cells and reprogramming. He completed his PhD with Professor Martin Pera (Monash University) and overseas postdoctoral training in Professor Peter Donovan's laboratory (University of California Irvine, USA) and subsequently Professor Minoru Ko's laboratory (National Institutes of Health, USA). His research in the past 12 years has led to improvement in methods of growing human pluripotent stem cells and generation of human induced pluripotent stem (iPS) cells. Recently Raymond joined the Centre for Eye Research Australia as a group leader of the Cellular Reprogramming Group and is mentored by Dr Alice Pébay. His current research focuses on using human iPS cells for disease modelling and harnessing their medical potential for eye research.

## *Dr Katharina Wystub-Lis* 1

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Katharina Wystub-Lis studied biology at the University of Giessen, Germany and received her Bachelor and Master degree in 2008 and 2010 respectively. In 2010, she started her PhD at the Max-Planck-Institute for Heart and Lung Research in Bad Nauheim, Germany within the department of Professor Thomas Braun under the supervision of PD Dr Thomas Boettger. Her work focused on the function of specific microRNAs and their role in cardiac differentiation

and function. Katharina received her doctoral degree in 2014 and worked as a postdoc until 2015 before joining the Harvey laboratory within the Developmental and Stem Cell Biology Division of the Victor Chang Cardiac Research Institute.

### *Dr Yinghong Zhou* 2

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Yinghong Zhou joined the Queensland University of Technology (QUT) in November 2009 where she completed her PhD on the interaction between donor cells and host cells during bone repair and regeneration. She is currently working as a postdoctoral researcher in the Bone Tissue Engineering Group at the Institute of Health and Biomedical Innovation (IHBI). Her research focuses on stem cell differentiation and biomaterial assessment for bone tissue engineering applications. Yinghong was appointed as the Coordinator of the Australia–China Centre for Tissue Engineering and Regenerative Medicine (ACCTERM) in 2013. ACCTERM was established to draw together new and longstanding research collaborations between China and Australia, and to aid in creating a highly visible hub for international collaborative research.

# *PRESENTATION ABSTRACTS*

## *Lgr5 stem cells and organoids in health and disease*

**Professor Hans Clevers**

Hans Clevers' laboratory has discovered novel ways to identify specialised stem cells in adult organs in mouse and man. Each of these stem cell types is responsible for renewal and repair of lost or damaged tissue, but can only do so for the tissue in which they reside. The group has also developed technologies to expand healthy and diseased stem cells in the laboratory for prolonged periods of time. Cultured stem cells form mini-versions of the organ from which they derive, so called organoids. These organoid technologies hold the future promise to replace organ transplantation. Currently, the group is already applying organoids derived from patients with cancer or hereditary diseases (such as cystic fibrosis) to study human disease in a dish. Moreover, patient-specific organoids are being used to test drugs and provide information on the best drug for the individual patient: precision medicine.

## *Exploring the boundaries of transcription factor-mediated reprogramming*

**Associate Professor Jose Polo**

One of the greatest promises of regenerative medicine is the theoretical ability to reprogram any cell type of the body into any other cell type. Classically two different approaches have been used for the generation patient-specific cell types: 1) induced pluripotency, which entails the reprogramming of adult cells into a pluripotent state (iPS cells), follow by the differentiation of the iPS cells to the desired target cell type and 2) transdifferentiation, which is the reprogramming of one adult cell type to another without traversing through the embryonic pluripotent state (ESC/iPS). Yet despite the great potential of these reprogramming approaches for cell-replacement therapies and diseases modelling, major hurdles delaying the clinical delivery of this promise are the fact that reprogramming processes (induced pluripotency and transdifferentiation) have inefficient discovery rates, are not well understood, and that differentiation of iPS cells into fully functional somatic cells is still a challenge. Only by addressing these problems at the root will the true potential of these TF reprogramming-based technologies be unleashed. Jose Polo will explore how unveiling the molecular mechanisms of these reprogramming processes will, in turn, push the limits of these technologies to generate new regenerative medicine strategies.

## *Modelling human fertility and reproduction with stem cells*

**Professor Amander Clark**

Six percent of the reproductive age population are infertile, one in one hundred women will enter menopause before the age of 40, one in thirty boys will be born with undescended testicles, and the incidence of testicular cancer in young adults is on the rise. What is causing these changes in human reproductive health? The answer in many cases

is unknown. We believe that stem cells can help scientists uncover causes of infertility, and in particular problems in developmental programming of cells that make up the reproductive tract. Amander Clark's laboratory focuses on the development of the human germline. The germline is the only cell type capable of passing the genome and a remodelled epigenome to future generations, making the germline central to reproductive health. The group studies mechanisms of human germline development using a cohort of human embryonic stem cell lines with sibling embryos documented to result in successful live births. Using this unique resource it uses directed differentiation to generate human germline cells in vitro to study mechanisms that promote germline specification and epigenetic reprogramming during prenatal life. It is anticipated that this model could be used in the future to understand effects of environmental agents and pharmaceuticals on a baby's germline during development in utero, and also to expand the differentiation portfolio to somatic cells of the ovary and testis towards regeneration of reproductive cell types to treat infertility.

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