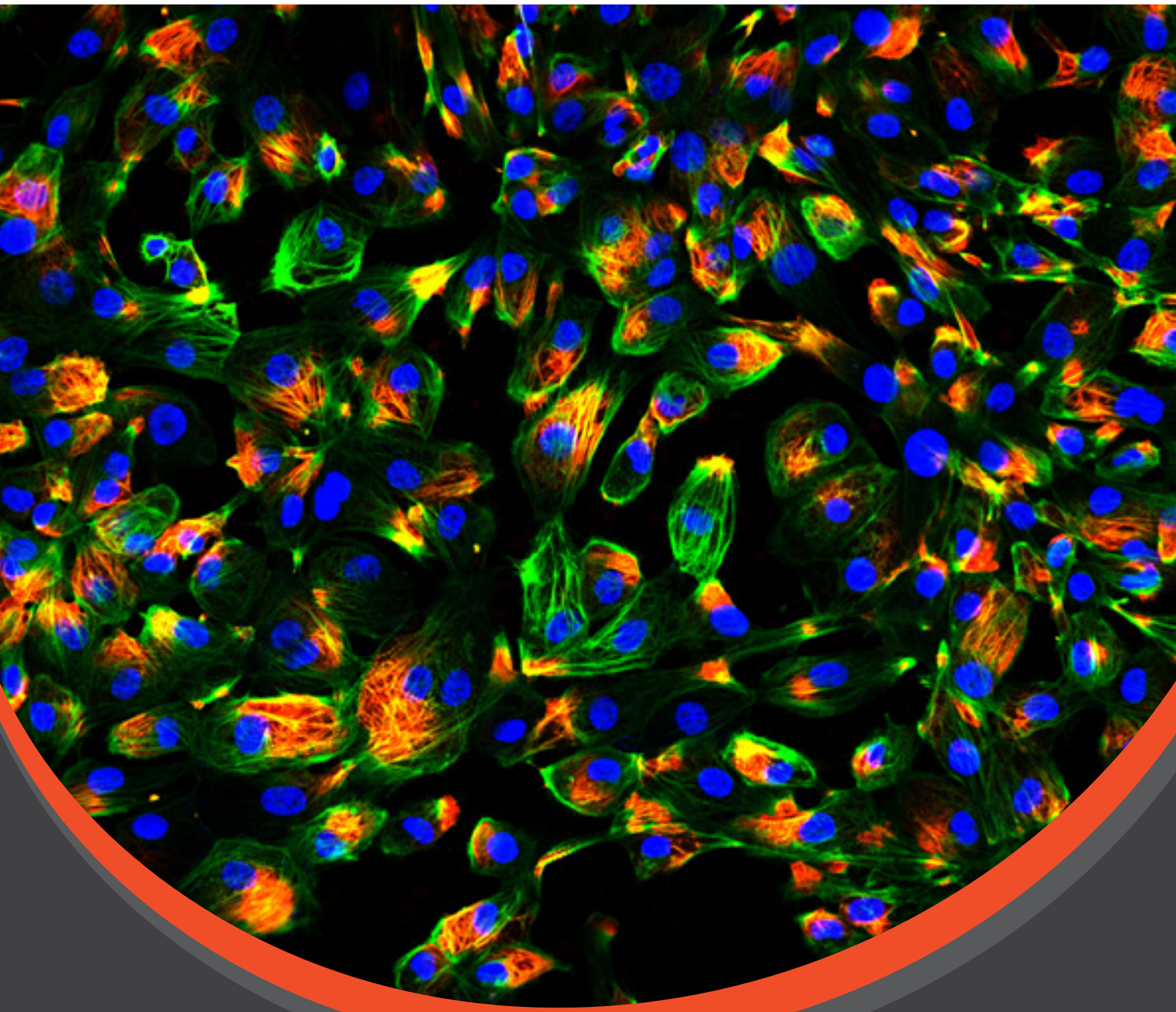
STEMCELLS® AUSTRALIA



Annual Report

2015

stemcellsaustralia.edu.au

"THE HIGHLY SKILLED SCIENTISTS FORMING THE RESEARCH TEAMS IN STEM CELLS AUSTRALIA ARE ENSURING THAT THIS COUNTRY WILL HAVE LEADERS IN THIS FIELD AND THAT THESE TECHNOLOGIES WILL BE AVAILABLE TO THE AUSTRALIAN PUBLIC WHEN THEY ARE NEEDED."

Professor David de Kretser
Chairman, Governance Committee

Supported by:

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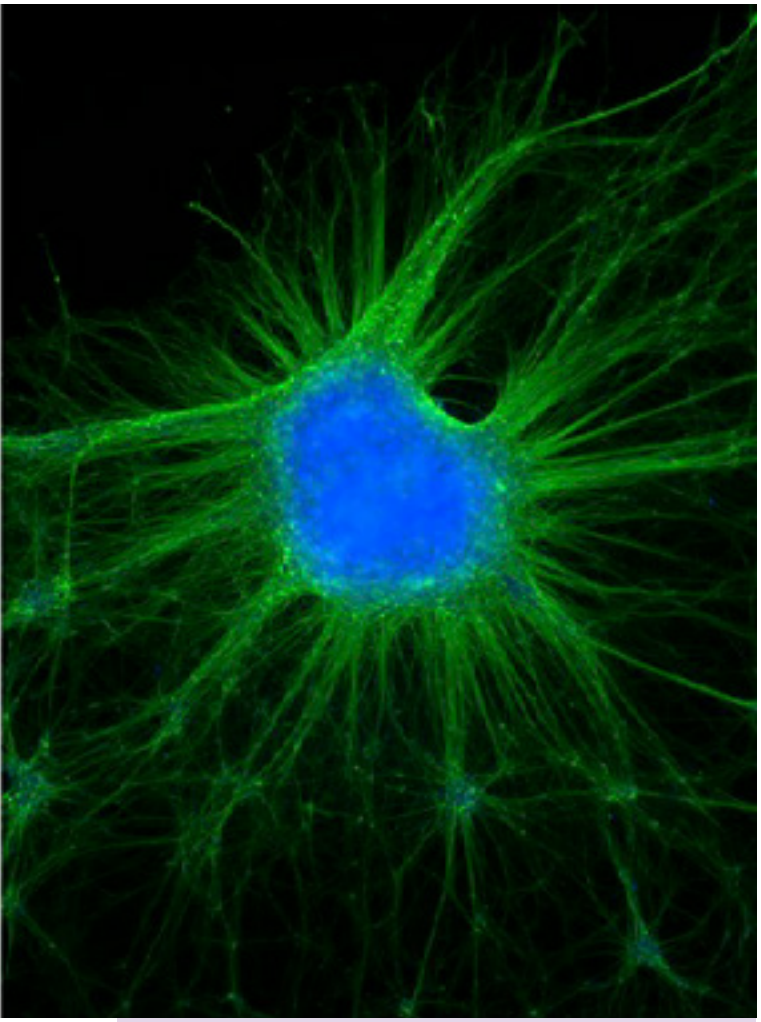
THE FLOREY

Cover Image: Human pluripotent stem cells differentiated into cardiomyocytes stained with DAPI (blue, nucleus), phalloidin (green, cytoskeleton), and α-actinin (red, sarcomere). Courtesy of Nathan Palpant (IMB,UQ)

Vision statement

To discover how to regulate stem cells in order to harness their potential for therapeutic purposes and to generate economically valuable biotechnologies.



Derived from human embryonic stem cells, this in vitro neuronal culture is approximately 6 weeks old and has formed large, dense nodes with interconnecting neural networks. Nuclei are stained with DAPI (blue); neural filaments are stained with BIII-tubulin (green). Courtesy of Jarmon Lees (UoM).

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Message From The Chairman

Into its fourth year, further significant advances have been made by scientists from Stem Cells Australia (SCA), a Special Research Initiative in stem cell sciences funded by the Australian Research Council (ARC).

The advances reported by the investigators highlight the value of a high level of cooperation and sharing of knowledge between Australian experts in the field, thereby enhancing their impact on stem cell research at both the National and International level.

The exploration of the fundamental mechanisms involved in stem cell regulation and differentiation are the focus of our initiative to enable the use of this knowledge for innovative biotechnological and therapeutic applications. Not only has this initiative supported excellence in stem cell research but it will also lead public debate and discussion about the important ethical, legal and societal issues associated with stem cell science. The unique multidisciplinary approach of this initiative has also nutured and trained the next generation of Australian stem cell scientists, strengthening Australia's future position in the field.

During 2015, our interdisciplinary research program has enhanced our understanding of the potential roles of stem cells in a range of conditions arising from the neuronal cell damage occurring from brain injury and the associated regeneration of neurons, to the effects of a heart attack which damages cardiac muscle requiring healing of the damaged tissue through the regeneration of new cardiac muscle cells and not the growth of scar tissue. It has also explored the use of new technology, CRISPR mediated genome editing, for modifying genes.

To promote interactions and information sharing between researchers and participating members, SCA held theme workshops which resulted in further cross disciplinary collaboration and greater research focus.

SCA core researchers were part of research teams that secured over $29 million in research funding in 2015 from various funding sources including ARC, National Health and Medical Research Council (NHMRC) and other national and international sources. Through the relationship with Canada's Centre for Commercialisation of Regenerative Medicine (CCRM), which facilitates protection of intellectual property and opens possibilities of future commercial developments, we were able to start the translation process.

In 2015, SCA used strategic funds to foster further collaboration between partners and provide the opportunity to bring in new collaborators to join our research community.

The expansion of our network progressed steadily in 2015 with a number of staff relocations. In 2015, we welcomed both Professor Peter Gunning (UNSW) in place of Professor Laurent Rivory and Professor Robyn Ward (UQ) in place of Professor Anton Middleberg to the Governance Committee.

The Commonwealth Scientific and Industrial Research Organisation (CSIRO) finalised their 5 year in-kind contribution, as a Partner Organisation, to our work. We are in the process of finalising a new Partner Organisation Agreement with Murdoch Childrens Research Institute (MCRI), to a joint research effort and to provide in-kind contributions for the remainder of the initiative.

Stem cell science is an extremely fast moving field of research with new breakthroughs being reported on an almost daily basis thereby increasing the likelihood of therapeutic developments. Our 2015 research publications have provided impact to the broader community through knowledge transfer in a number of key areas as we gain further understanding of cell differentiation. The collaborative effort of scientists in SCA places the Australian scientific community at the world's leading edge of research in this important field.

Our scientists have also enhanced public knowledge of stem cells and their future potential impact of various branches of biomedical science. In this manner SCA is supporting Prime Minister Turnbull's call for scientific endeavour to underpin the development of novel technologies and industries.

It is a privilege to be associated with this ground breaking field of medical research, with the potential to transform the lives of many people affected by all too common medical conditions prevailing in modern society.

Professor David de Kretser
Chairman, Governance Committee

Message From The Program Leader

In 2015 Stem Cells Australia passed the halfway mark in its term as an ARC Special Research Initiative.

The year began with a resounding endorsement of our efforts from the ARC Interim Review of SCA, and a renewal of our funding through to 2018. The ARC Review Panel also provided a number of very constructive suggestions, in the areas of education, sustainability planning and other key functions, which we are now implementing through our Strategic Plan.

Some of the new developments include the appointment of an Education Officer, Dr. Toby Merson, to oversee educational activities across our network, and the establishment of a Strategic Planning Working Group, which includes a number of mid-career scientists, to look into new mechanisms for sustaining a coordinated national program in stem cell sciences and regenerative medicine beyond 2018.

We were delighted to learn this year that our combined efforts with the Australasian Society for Stem Cell Research (ASSCR) to bring the annual meeting of the International Society for Stem Cell Research (ISSCR) to Melbourne in 2018 were successful.

The ISSCR meeting is the premier annual event in the field, and the Society's decision to come to Melbourne is a great endorsement of the quality and international profile of science here. The combined SCA Retreat/ASSCR annual meeting events over the past two years have helped to unite our research community, and undoubtedly contributed to the success of the bid.

SCA also was successful in a solicited bid to Bioplatforms Australia (BPA) to develop a stem cell database. The project will exploit BPAs multiple -omics capacity to study well-defined stem cell populations at the level of the transcriptome, proteome and metabolome. The program includes a significant component of single cell analyses, an exciting area of contemporary stem cell research. The BPA support for this program provides up to $1million equivalent in research service provision for the project. We anticipate that this program will result not only in a great resource for the international community but also in some important discoveries and some very high profile research outputs.

A number of SCA researchers received some very impressive honours and recognition this year, including Perry Bartlett (2015 CSL Florey Medal, Lifetime Achievement Award from Research Australia); Christine Wells and Ryan Lister (Metcalf Prize); Nadia Rosenthal and Melissa Little (induction into the Australian Academy of Medical Science); Peter Currie (Eureka Prize); Jana Vukovic (ARC Discovery Early Career Research Award); Anja Knaupp (NHMRC Peter Doherty Early Career Fellowship); Martin Pera (election to the Board of the ISSCR as Clerk of the Society).

Success begins with scientific excellence, and this year as in the past, publications from SCA researchers exemplified the research strengths that underlie our consortium.

I will just name a few highlights here. Melissa Little's study in Nature describing kidney organogenesis in a dish was a stunning example of self-organising morphogenesis in vitro. The work is a real tribute to Melissa's persistence in this field over many years.

Richard Harvey and his co-workers described a signalling pathway that can trigger cardiomyocyte proliferation and heart regeneration in Nature Cell Biology. Dhanisha Jhaveri, Perry Bartlett and their colleagues discovered that there are two classes of stem cell in the adult hippocampus (reported in the Journal of Neuroscience), opening up the way for novel insights into the physiological significance of adult neurogenesis in memory and learning. Finally Christine Wells and Ernst Wolvetang contributed to a large RIKEN consortium study published in Science that uncovered new pathways of gene regulation in mammalian cells.

Inevitably, over the course of a seven-year program, some of our scientists will embark on their own new journeys. This year Nadia Rosenthal left Australia to take up a new position as Scientific Director of the Jackson Laboratory. Nadia was one of the key founders of SCA. She served on our Scientific Leadership Group, and was a great advocate of our young investigators. We will miss her leadership, collegiality, and insight, but do we recognise what a great opportunity her new post is for her. We will of course keep in touch and look for opportunities to continue to work with her.

My thanks to all of our scientists, our affiliates, our administrative staff, our Scientific Advisory Board and Governance Committee, our member organisations, and the ARC for keeping our efforts on the leading edge of stem cell research.

Professor Martin Pera
Program Leader

Program Highlights

SCA researchers and students continue to advance knowledge in the area of stem cells, through world leading research and collaborations, presentations at international meetings, publications in high impact journals and securing external funding.

An important highlight was the announcement by the ISSCR that their 2018 Annual Meeting will be held in Melbourne, an honour for the Australian research community. SCA was a member of the successful consortium behind the bid, led by the ASSCR, and included the Melbourne Convention and Visitors Bureau together with support from industry, government and the scientific and health communities.

The ISSCR annual meeting is the premier international society for stem cell science, providing an opportunity for approximately 4,000 scientists, clinicians, educators and industry professionals from over 55 countries to share new data, learn from peers and discover global advances within the stem cell field.

Destination Melbourne for international stem cell community in 2018

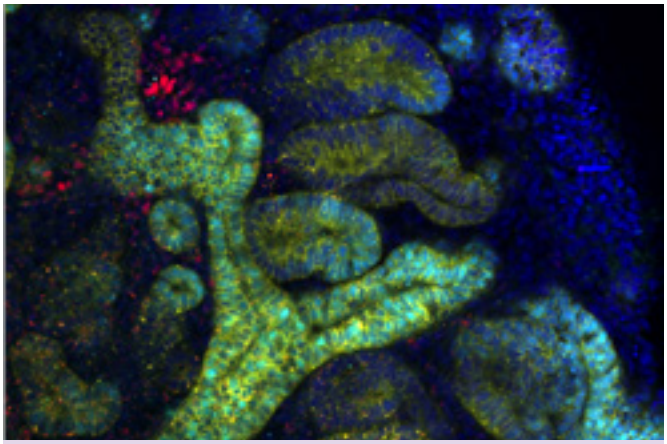
Research Performance

Mini kidneys from stem cells

Professor Melissa Little and her colleagues have perfected a method of turning stem cells into a mini-kidney model for use in drug screening, disease modelling and cell therapy.

By building on their research from 2013, the researchers from MCRI, the University of Melbourne (UoM), the University of Queensland (UQ) and Leiden University Medical Centre in the Netherlands have been able to grow mini-kidney organoids in a dish, which contain all the different cell types (more than 20) normally present in the human kidney. It is a model system that can be used to study the human kidney, but at only 2mm in size is a fraction of the size of a mature adult kidney.

Key to this breakthrough was adding different concentrations of growth factors at various times, allowing researchers to mimic normal development. The mini-kidney that the team produced is similar to the kidney in a developing foetus, as it contains all the different cell types at their very early developmental stage. They will now optimise this process to vary the amount of each cell type present. The breakthrough could allow the use of mini-organs to screen drugs either for the treatment of kidney disease or to find out if a new drug is likely to injure the kidney. The study was published in *Nature* (2015) vol 526(7574), 564-568.



Kidney organoid generated from human iPS cells. The image shows a kidney organoid containing two kidney progenitors, the ureteric tree (yellow with cyan) and nephron progenitor (red), as well as developing nephrons (yellow). *Courtesy of Minoru Takasato (MCRI).*

"The mini-kidney we have been able to grow, is very complex and more like the real organ. This is important for drug testing as we hope they will respond to the drugs as a normal organ might," Professor Little said.

Different populations of cells in the brain may regulate mood and memory

Queensland Brain Institute (QBI) researchers at UQ are one step closer to understanding how the brain regulates memory and mood, thanks to the discovery of two distinct types of stem cells that give rise to new neurons in a region of the brain known as the hippocampus.

Dr Dhanisha Jhaveri. Courtesy QBI (UQ)

QBI's Dr Dhanisha Jhaveri, the study's lead author, said researchers had isolated pure populations of these cells for the first time. The discovery may have implications for the treatment of learning and mood related disorders. Dr Jhaveri's work made the cover of the *Journal of Neuroscience* (2015), vol 35(21), 8132-8144.

Heart regeneration after heart attack

Scientists have discovered a way to stimulate muscle cell growth in the heart, limiting the damage to this vital organ after a heart attack. It is hoped that the exciting research, which was conducted by Professor Richard Harvey at the Victor Chang Cardiac Research Institute (VCCRI) in collaboration with Professor Eldad Tzahor from the Weizmann Institute of Science in Israel, could help the 55,000 Australians who suffer a heart attack each year.

"SCIENTISTS HAVE NOW FOUND A NEW WAY TO POTENTIALLY REGENERATE THE HEART AFTER A HEART ATTACK BY REPLACING LOST MUSCLE CELLS.."

Scientists have now found a new way to potentially regenerate the heart after a heart attack by replacing lost muscle cells. The scientists wanted to find a better way to stimulate the regeneration of heart muscle cells in adults, to improve recovery after a heart attack.

Previous studies have demonstrated that it is possible to coax heart muscle cells to proliferate again, but only at very trivial levels. In this most recent work, the research team have been able to increase heart muscle cell numbers by an impressive 45% after a heart attack. *Nature Cell Biology* (2015), vol 17(5), 627-638.

Unravelling the biological mystery of how cells regulate their fate

Professors Christine Wells and Ernst Wolvetang from UQ were part of an international consortium that has been able to shed light on how mammalian cells transition from one cell type to another during development and in response to stress or infection.

This landmark study published in the prestigious journal *Science* (2015), 347(6225), 1010-1014, uncovers striking new features of gene regulation in mammalian cells. In order to conduct this study, scientists from 114 institutes in more than 20 countries joined forces. This work builds on previous discoveries made by the Japanese-led Functional Annotation of the Mammalian Genome (FANTOM5) consortium.

fantom.gsc.riken.jp.

Researchers were able to show the importance of a key regulatory "switch" involved in triggering a cascade of genetic changes that ultimately result in dramatic change in the attributes of the cell.

While scientists have long recognized that activation of particular genes in a cell were controlled by two different mechanisms encoded in the cell's DNA, known as enhancers and promoters, exactly how these molecular tools influenced gene expression remained unclear.

By conducting a comprehensive analysis of many different types of human and mouse cells, researchers were able to show that it is the enhancers regions of DNA, often located a long way from the actual genes of interest, that were crucial to coordinating rapid changes in gene expression. Enhancer based transcription is an essential first phase of gene activation when stem cells differentiate. The results of this study will change our understanding of how stem cells transition into new cell types, and will help us to understand disorders of human development.

Reputation Building

Our researchers continued to build and strengthen their reputation in 2015 with

- One new patent being filed with the US patent office by Professor Richard Harvey's group at VCCRI and Weizmann Institute, covering the role of Erbb2 in cardiomyocyte proliferation.
- 113 articles in open access and peer reviewed journals exceeding the 2015 KPI target of 90 journal publications. Articles were published in a varied collection of high impact factor (IF) journals such as
  - *Blood*
  - *Cell Stem Cell*
  - *Cell*
  - *Cell Metabolism*
  - *Journal of the American College of Cardiology*
  - *Nature*
  - *Nature Cell Biology*
  - *Nature Biotechnology*
  - *Nature Methods*
  - *Nature Communications*
  - *Science*

Of the published articles

- 100 articles where published in peer-reviewed journals
- 4% of articles published in journals with an IF ≥ 40
- 20% of articles published in journals with an IF > 10, exceeding the 2015 KPI target of 15%
- Over 73% articles published with collaboration with another partner organisation, national or international organisation

The work carried out by SCA researchers including students have been once again acknowledged and showcased globally with

- Over 67 invited presentation at international conferences/meetings
- Over 30 oral presentations and 30 poster presentations at conferences/meetings

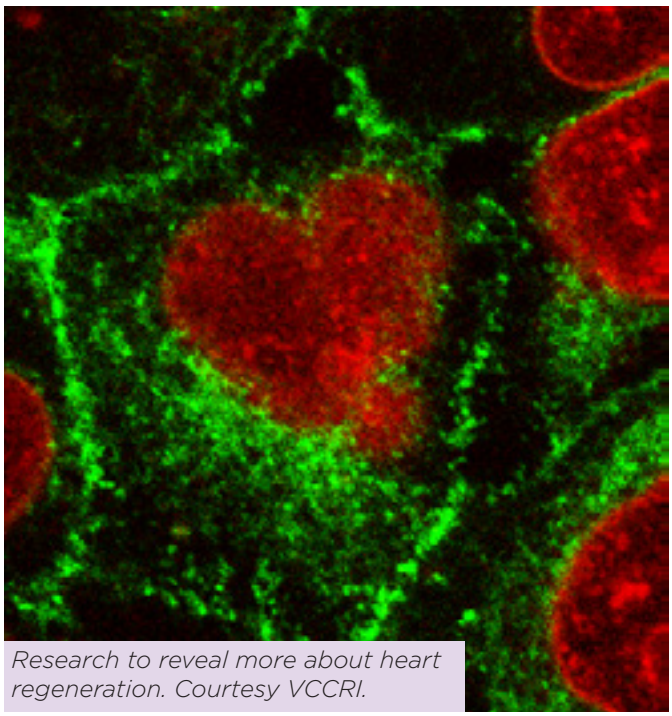
SCA researchers participated at a global level through inclusion on advisory boards and various committee memberships, panellists, conference organisers and session chairs and delivering public lectures. Program Leader Martin Pera was elected to the Board of the International Society for Stem Cell Research and will serve as Clerk of the Society.

Funding and Recognition

Multimillion Dollar International Grants

Australian Institute for Bioengineering and Nanotechnology (AIBN) researcher Professor Lars Nielsen at UQ was the recipient of the multi-million dollar grant, The Novo Nordisk Foundation (NNF) Laureate Research Grant which will provide $8.6 million from 2015 over seven years to crack a 90-year old mystery around the detailed biology of cancer cells, to develop complex computational models of cell metabolism.

The research aims to understand why cancer cells and other fast-growing cells produce lactate, which could lead to better cancer therapies.



Research to reveal more about heart regeneration. Courtesy VCCRI.

Professors Robert Graham and Richard Harvey from the VCCRI are part of an international research collaboration awarded a prestigious grant from the Fondation Leducq (US$ 6 million from 2016 over five years) to improve our understanding and treatment of cardiovascular disease.

Despite more than a decade of stem cell research that initially appeared to be very promising in this regard there is currently no therapy that successfully regenerates heart tissue. One new promising line of research that this collaboration will explore is the heart's limited ability to produce new muscle cells.

Professor Trevor Kilpatrick, from UoM and the Florey Institute of Neuroscience and Mental Health (The Florey), has been awarded a prestigious research grant from the American National MS Society (NMSS). The grant was announced as part of the 2015 NMSS funding round which awarded US$28 million across 84 new research projects and training awards. Professor Kilpatrick's project will look at the role of a specific gene called MERTK in developing Multiple Sclerosis. Importantly; this study will provide a model for future studies of the effects of single genes in MS.

Furthermore SCA researchers have been successful in securing over 20 new grants totalling over $29 million to begin in 2015, of which over $7 million is to be used in the year 2015 and over $1million in philanthropic donations.

SCA researchers also received fellowships, inductions and other prestigious awards in recognition and acknowledgement to their excellence in their area of expertise, these included

Inductions and Fellowships

Alexandra Harvey (UoM) - Melbourne Research (Career Interruption) Fellowship

Anja Knaupp (Monash) - NHMRC Peter Doherty Early Career Fellowship

Melissa Little (MCRI) – Fellow of the Australian Academy of Health and Medical Sciences

Melissa Little (MCRI) - Boerhaave Professorship, The Netherlands

Nadia Rosenthal (Monash) - Fellow of the Academy of Medical Sciences, UK

Nadia Rosenthal (Monash) - Fellow of the Australian Academy of Health and Medical Sciences

Pankaj Sah (QBI, UQ) – Appointed as Director of QBI.

Ann Turnley (UoM) - NHMRC Research Fellowship Extension

Jana Vukovic (UQ) - ARC Discovery Early Career Research Award

The 2015 NSCFA Metcalf prize recipients Professors Christine Wells and Ryan Lister

Awards

Perry Bartlett (UQ) - 2015 CSL Florey Medal

Perry Bartlett (UQ) - Lifetime Achievement award from peak body, Research Australia

Justin Cooper-White (UQ) - 2015 Aon Risk Solutions Regenerative Medicine Award for his research at the interface of engineering and biology

Peter Currie and Team (Monash) - Eureka Prize for Scientific Research

Katherine Gill (UoM) - Harold Mitchell Foundation Travel Award

Elizabeth Mason (UQ) - Donald Tugby Prize in Nanotechnology

Enzo Porrello (UQ) - UQ Foundation for Research Excellence Award

Christine Wells (UQ) - National Stem Cell Foundation of Australia Metcalf prize in recognition of contribution and leadership in stem cell research

Ryan Lister (UWA) – National Stem Cell Foundation of Australia Metcalf prize in recognition of contribution and leadership in stem cell research

Ernst Wolvetang (UQ) - 2014 LSQ Regenerative Medicine Prize

Perry Bartlett (UQ) - 2015 CSL Florey Medal.

Exercise the best way to 'get the game started'

Twenty years ago Professor Perry Bartlett made the ground breaking discovery that the human brain can change and regenerate. More recently he and his team at the QBI have reversed dementia in mice through exercise, and are now hoping they can do the same in humans.

For that discovery and his work in the field of neurology, Professor Bartlett was honoured with one of Australian science's biggest prizes, the CSL Florey Medal, awarded once every two years for major biomedical and health advancements.

Capacity Building and Education

SCA has an ongoing commitment to developing future researchers and ensuring the brightest minds are nourished, supported and retained. There were a total of 65 postdoctoral researchers, out of which 14 were new postdoctoral researchers; and 57 postgraduate students, out of which eight were new postgraduate students and nine completing their PhD studies.

Postgraduate students and early career researchers from each of the partner organisations had the opportunity to enhance their skills in many aspects throughout their candidature or employment, through many of the training and workshops offered by the respective organisation.

Four new Affiliate Investigators were welcomed to the initiative. They were Professor Peter Currie from Monash University and the Australian Regenerative Medicine Institute (ARMI), Dr James Hudson from UQ, Dr Shalin Naik from the Walter and Eliza Hall Institute of Medical Research (WEHI) and Professor Ryan Lister from University of Western Australia.

Dr Tobias Merson was appointed as Education Officer, to co-ordinate student training efforts across the initiative. He will provide high-level integration of education and coordination of training opportunities, by facilitating inter-lab transfers for SCA students and postdoctoral researchers to learn new experimental techniques and technologies.

Theme meetings were held throughout the year for each of the four themes.

We organised and hosted a full day workshop *Taking Your Research to Commercialisation* at the University of Melbourne. The workshop was aimed at broader training in the form of Intellectual Property management to SCA members and the broader university faculty. Key speakers included:

- Dr Doran Ben-Meir – Executive Director, Research, Innovation and Commercialisation, UoM
- Mr Ken Seidenman – Overseas Qualified Attorney, FB Rice
- Dr Chris Behrenbruch – CEO Irukandji Capital and Vice Chancellor's professorial Fellow, Monash University
- Dr John Kurek – Investment Manager, Uniseed Ventures, UoM
- Professor Gary Anderson – Director Lung Health Research Centre, UoM
- Associate Professor Vern Bowles – Hatchtech Founder and Deputy Director of Centre for Animal Biotechnology, UoM

Throughout the year several public and special forums on stem cells were held in various venues in Melbourne and Queensland aimed at informing and educating the broader community and featuring local and international experts. Discussion titles included:

- *Hype, Hope or Reality: Can we make eggs or sperm from stem cells?*
- *Made to Order: Can science regenerate body parts?*
- *Selling Stem Cells: The need to reconcile hype, hope and evidence*
- *Bodies, Borders and Biologicals: Ethical considerations of medical tourism*
- *Brainoids: Growing brains in a dish*
- *Translating Stem Cell Research Into Real Health and Economic Benefits*

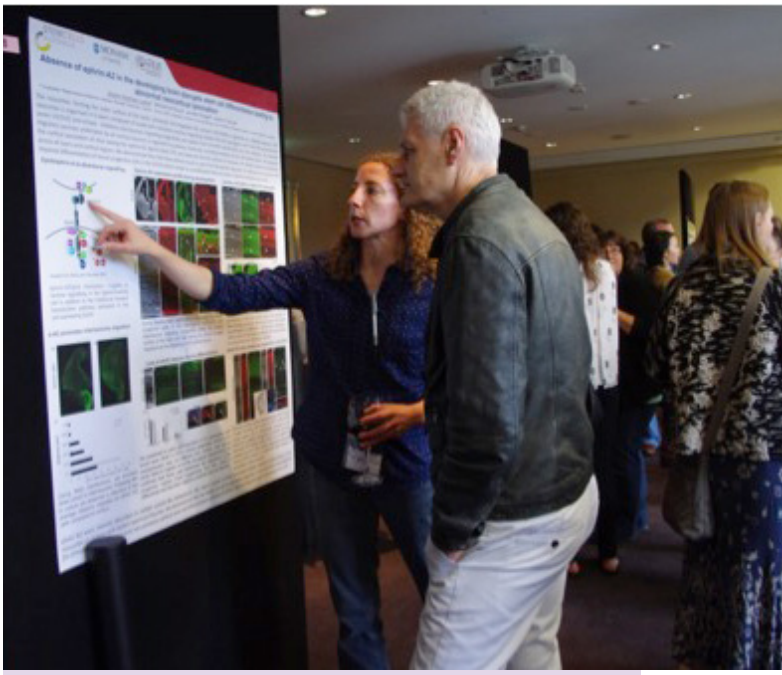
Networking

For the second consecutive year SCA partnered with ASSCR to hold a joint conference, incorporating the SCA Annual Retreat, and bringing together scientists from basic and clinical research, engineering and industry. Over 115 members of the SCA network including researchers, students, members of the Scientific Advisory Committee and Governance Committee attended our 2015 SCA Annual Retreat.

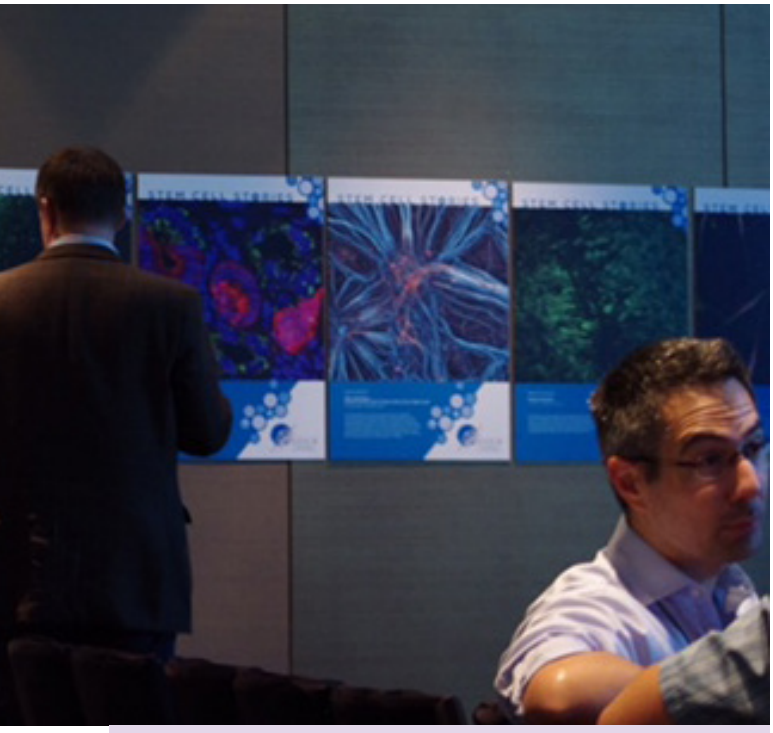
The meeting also coincided with the launch of the *Nature Regenerative Medicine* journal of which Professor Nadia Rosenthal is the inaugural Editor-in-Chief.

Following a successful review of a proposal to Bioplatforms Australia (BPA), SCA was selected to carry out a collaborative project aimed at the establishment of a stem cell database. The project will use the multiple -omics capability of BPA to study well defined stem cell populations, and will include a strong component of single cell analysis. BPA will be offering the equivalent of up to $1 million in research service provision to support this exciting scientific venture.

In conjunction with AusBiotech, SCA co-hosted a one day Regenerative Medicine Symposium featuring Professor Masayo Takahashi from Japan discussing her clinical research in macular degeneration.



Professor Ed Stanley and Dr Jihane Homman-Ludiye at the 2015 SCA Retreat in the Hunter Valley.



The Australasian Society for Stem Cell Research displayed the 'Stem Cell Stories' image exhibition at the 2015 SCA joint conference in the Hunter Valley.

During 2015, Stem Cells Australia also hosted visits by leading international researchers including

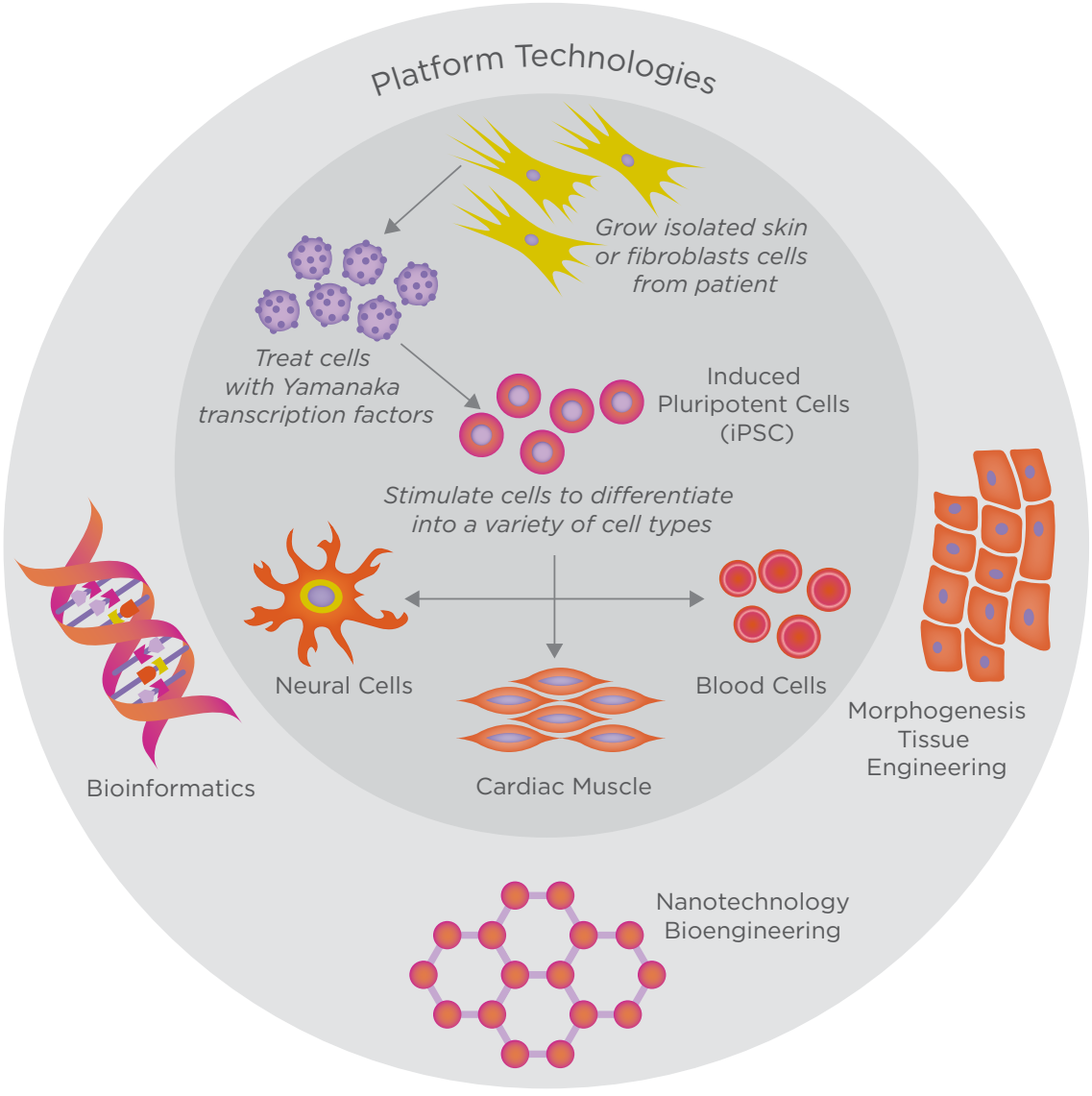
- Dr Kouichi Hasegawa, Institute for Integrated Cell-Material Science (iCeMS), Kyoto University, Japan
- Professor Jacob Hanna, Weizmann Institute of Science, Israel
- Professor Jeanne Loring, Centre for Regenerative Medicine, The Scripps Research Institute, La Jolla, USA
- Professor Timothy Caulfield, Faculty of Law and the School of Public Health, University of Alberta, Canada
- Professor Jeremy Sugarman, Johns Hopkins Berman Institute of Bioethics, Baltimore, USA

Overseas students Eline Dirven from Lieden University, Netherlands, and Danila Vittori from Rome each spent over 14 weeks and six months respectively undertaking stem cells research and training with SCA researchers.

Research Program

Stem cells have the capability to self-renew (to divide to produce more stem cells) or to undergo differentiation into specialised cell types. Thus stem cells can provide a renewable source for replacement of senescent, dead or damaged cells in tissues.

SCA scientists study both pluripotent stem cells and tissue stem cells. Pluripotent stem cells, like cells of the early embryo, can turn into any type of tissue cell. Understanding how pluripotent stem cells chose between self-renewal and a specific direction or differentiation is a key focus of SCA research, because this information is critical to our ability to form specialised tissues from pluripotent cells. SCA scientists also study stem cells in the heart, brain and in the blood forming system. Here the goal is to understand the role of these stem cells in tissue maintenance and regeneration and to exploit the findings to enhance the innate ability of our organs to undergo repair after injury or during disease. These biological discovery themes are supported by platform technologies in bioinformatics, nanotechnology, bioengineering and tissue morphogenesis.



Key biological questions for each theme

Pluripotency and Reprogramming

1. Understand how to assess and ensure the quality of cellular reprogramming; the process of converting adult cells back to the embryonic state.

2. Discover novel networks controlling pluripotency and self-renewal.

3. Generate functional specialised cells from pluripotent stem cells (cardiac, neural and blood lineages).

Neural Regeneration and Repair

1. Determine the factors regulating endogenous neural precursor maintenance and differentiation in health and disease.

2. Define how to produce specific neural phenotypes from in-vitro-generated stem cells.

3. Understand the function of neural precursor progeny in the central nervous system (CNS).

Cardiac Regeneration and Repair

1. Investigate how capacity for regeneration is maintained in the heart, and how can it be rejuvenated in aging and disease.

2. Define the molecular underpinnings of cardiac repair.

3. Determine whether molecular switches underlie cell cycle re-entry of adult cardiomyocyte (CM) in mammals vs more regenerative vertebrates.

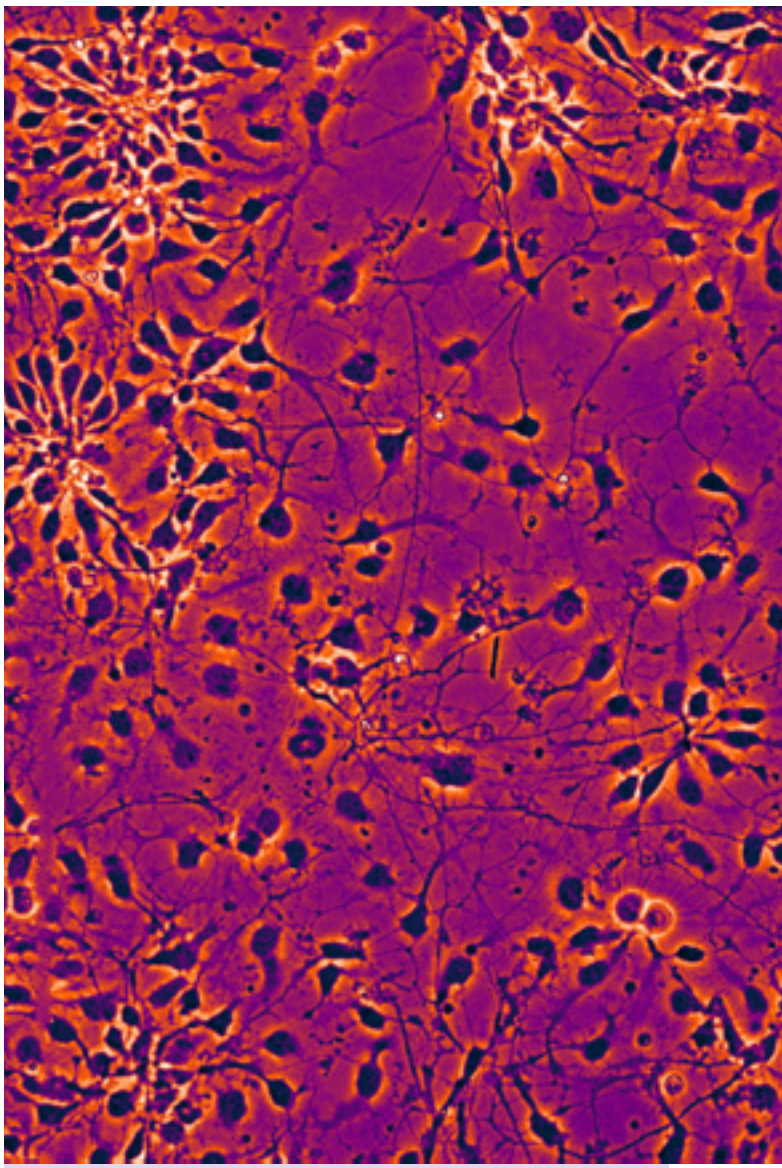
Haematopoiesis

1. Understand the molecular mechanisms controlling specification of haematopoietic stem cells (HSC) during development.

2. Determine the molecular interventions required to generate a new source of HSC from either pluripotent cells or mature blood cells.

3. Investigate whether mathematical models adequately define cell differentiation and transcriptional regulatory networks.

4. Determine whether HSC derived by cell reprogramming strategies exhibit identical functionality to their adult counterparts.



Neural Henna captures the beauty of neural stem cell differentiation. The neurons cluster together forming spectacular neural rosettes, so called because of their striking similarity to flower blossoms. This in vitro human embryonic stem cells derived neuronal culture is approximately 3 weeks old. Courtesy of Jarmon Lees (UoM).

Theme: Pluripotency and Reprogramming

Professor Martin Pera and **Professor Peter Gray**

Revolutionised Prospects for Regenerative Medicine and Cell Replacement in the Body

Human Embryonic Stem Cells (ESC) and more recently, adult somatic cells reprogrammed to a stem cell state (iPSC) have transformed our capacity to probe developmental and disease mechanisms, and have revolutionized prospects for regenerative medicine and cell replacement in the body.

This research program aims to understand the regulation of the growth and differentiation of pluripotent stem cells. The theme has three main focus areas:

- Propagation and maintenance of pluripotent stem cells
- Cellular Reprogramming
- Differentiation of pluripotent stem cells

At the end of 2015, cellular therapies derived from pluripotent stem cells were undergoing clinical trials for macular degeneration (a very common cause of blindness), spinal cord injury, Type I diabetes, and heart failure.

The rapid growth in the use of pluripotent stem cells in functional genomics, disease modeling, and drug discovery, suggests that they will soon become an integral part of the basic armamentarium of biomedical research, along with animal transgenes, -omics, imaging, and other essential platform technologies.

This year scientists in the pluripotency program achieved a number of breakthroughs in the area of stem cell differentiation. Melissa Little published a seminal work in *Nature* on kidney morphogenesis in vitro. The stunning images in this paper offer a glimpse into the future for the generation and use of stem cell derived organoid structures.

Christine Wells and Ernst Wolvetang contributed to the generation and analysis of a molecular roadmap of neural differentiation, published by the FANTOM consortium in Science. The study provided exciting new insights into mammalian gene regulation.

Mirella Dottori and her co-workers identified a novel caudal neural progenitor that displays an interesting degree of developmental plasticity.

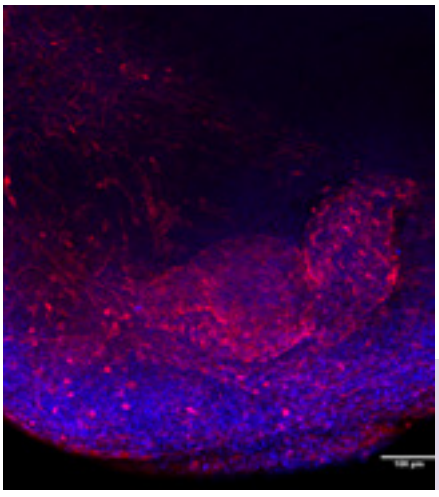
> "THE RAPID GROWTH IN THE USE OF PLURIPOTENT STEM CELLS IN FUNCTIONAL GENOMICS, DISEASE MODELING, AND DRUG DISCOVERY, SUGGESTED THAT THEY WILL SOON BECOME AN INTEGRAL PART OF THE BASIC ARMAMENTARIUM OF BIOMEDICAL RESEARCH..."

David Elliott, Richard Harvey, Andrew Elefanty and Ed Stanley contributed to a total of five studies of the production of cardiomyocytes from pluripotent stem cells, their use in studying cardiac electrophysiology, and technology for tracking these cells in vivo.

Through a longstanding collaboration with Gordon Keller in Toronto, Elefanty and Stanley published studies on the origin of hemogenic and vascular endothelium, and the generation of pancreatic progenitors.

Though the use of embryos for cell line generation is no longer so much at the forefront of the field, controversies around the application of pluripotent stem cells in research and medicine continue to arise. Martin Pera and Megan Munsie, along with Scientific Advisory Board members Christine Mummery and Patrick Tam and colleagues, published a commentary on formation of embryo-like structures in vitro from pluripotent stem cells. New developments in this and other arenas may prompt a review of current regulations, and SCA will contribute actively to informing the public about the science behind the headlines.

In 2016, as we begin strategic discussions concerning the future of national efforts in stem cell research after the term of SCA funding, we will be working hard to focus the attention of the public, private and philanthropic sectors on the clinical advance of pluripotent stem cell derived therapies, and the strategic role that the pluripotent stem cell platform will play in the future of Australian biomedical research.



Neural progenitors derived from human pluripotent stem cells. Courtesy of Claire Cuddy and Anna Lauxen (UoM).

Theme: Neural Regeneration and Repair

Professor Perry Bartlett and **Professor Trevor Kilpatrick**

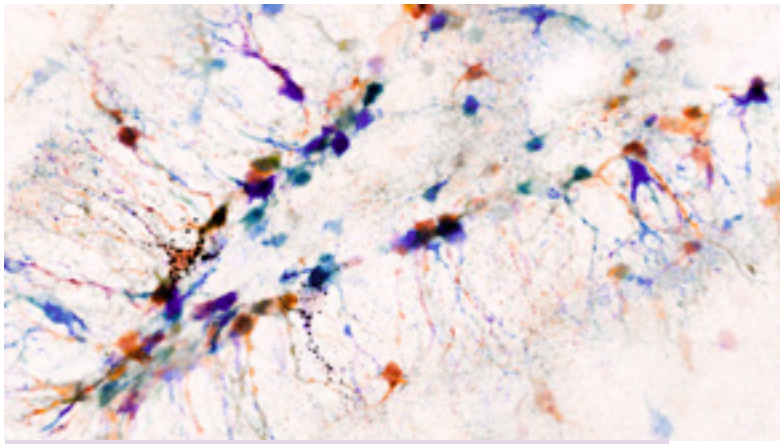
Brain Cell Regeneration and Repair

This theme explores the regulation of neural cell regeneration within the CNS. A key goal is to identify and characterise functionally distinct subpopulations of neural stem and progenitor cells within the developing and adult brain.

Using molecular analyses that define and segregate populations based on their unique molecular signatures, we are examining the properties of each population under the normal and pathological conditions to better understand how the CNS is generated and how it is remodeled throughout adult life.

Research projects involve understanding molecular and cellular mechanisms regulating the activity of endogenous populations of neural precursor cells, as well as generating different neuronal subtypes from ESC and iPSC sources.

For the first time we have demonstrated that the adult hippocampus contains distinct populations of quiescent stem cells that are activated by different stimuli to generate new neurons. In addition, we have shown that the neuronal progeny of these different classes of stem cells display distinct receptor phenotypes reinforcing our hypothesis that these distinct stem cell progeny may play different roles in regulating hippocampal functions such as cognition and mood.



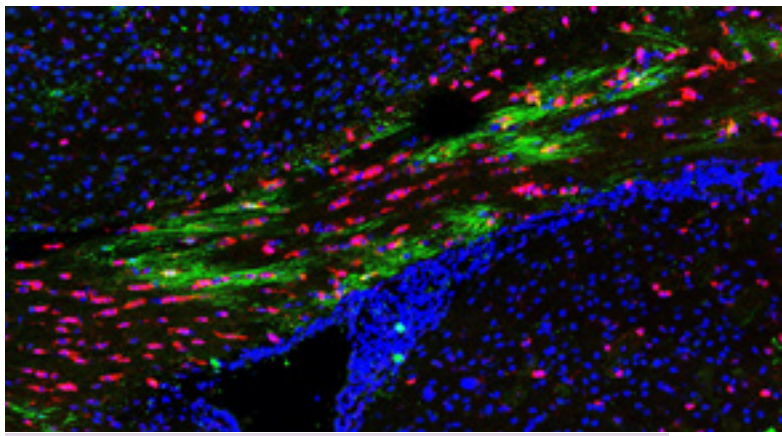
The image identifies the 'real' stem cells in the hippocampus of an adult mouse by the presence of epidermal growth factor receptors (black puncta) on the surface of the Nestin-GFP cells. Courtesy of Dhanisha Jhaveri (QBI, UQ).

"OUR RECENT RESEARCH DEMONSTRATES THAT NEURONAL ACTIVITY REGULATES MYELINATION IN THE CENTRAL NERVOUS SYSTEM IDENTIFYING ACTIVITY-DEPENDENT MYELINATION AS A NOVEL FORM OF NEUROPLASTICITY"

Another major advance has been the purification of the hippocampal stem cell to near homogeneity, allowing us to directly interrogate the phenotype and regulation of these stem cells. We have shown by fluorescent activated cell sorting that the hippocampal stem cell expresses both EGF receptor and Nestin allowing us, for the first time, to positively identify this cell in situ, as shown below.

The theme also explores the cellular and molecular mechanisms that underlie the generation and regeneration of myelin in the CNS. A key objective is to dissect the molecular code that instructs neural stem and progenitor cells to produce new myelinating oligodendrocytes. To achieve this, we are using sophisticated transgenic tools and next generation RNA sequencing to define the transcriptional network encoding remyelination mediated by distinct populations of neural stem and progenitor cells. We are also investigating how electrical activity within axons triggers them to become myelinated.

The knowledge gained will instruct the development of a new class of pro-myelinating drugs to treat demyelinating diseases such as multiple sclerosis.



Segments of newly-synthesised myelin (green) are produced by oligodendrocytes within the white matter of the mouse brain in response to heightened neuronal activity. Activity-dependent myelination is believed to facilitate the learning of complex motor tasks. The cytoplasm of mature oligodendrocytes is revealed with an antibody against CC1 (red), nuclei are labelled with Hoechst DNA dye (blue). Courtesy of Stan Mitew (The Florey).

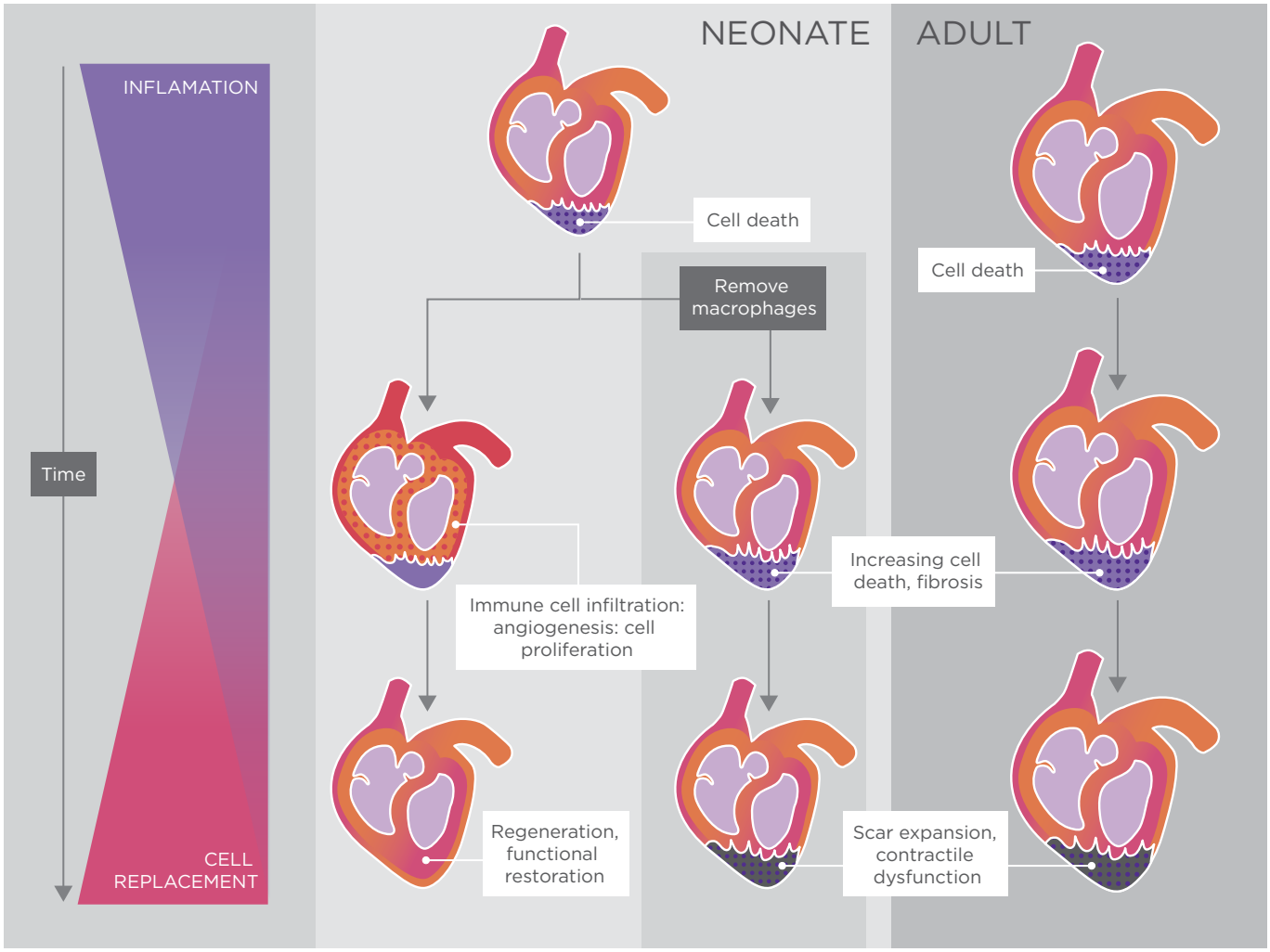
Theme: Cardiac Regeneration and Repair

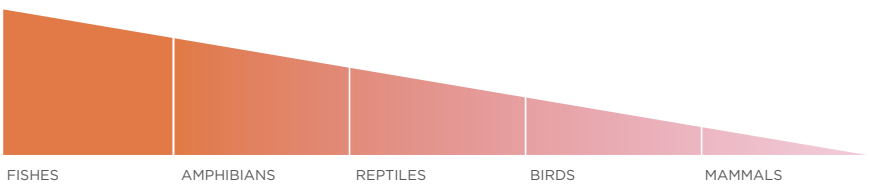
Professor Nadia Rosenthal and **Professor Richard Harvey**

Regeneration and Repair of Diseased or Injured Heart Tissue

This theme aims to develop a broad picture of the cellular and molecular basis of stem and stromal cell populations in the adult heart in health and disease, including their origins, hierarchies, paracrine functions and regenerative potentials. Furthermore, it seeks to understand the biochemical and epigenetic basis of CM proliferation and cell cycle withdrawal as crucial additional targets of regeneration therapies.

Cardiac Injury Response





A schematic of the cellular regenerative responses to cardiac injury in different species. Courtesy of Nadia Rosenthal (ARMI, Monash University).

Characterisation of the cellular processes that underlie cardiac regeneration in permissive models such as the zebrafish, as well as in periods of augmented cardiovascular repair in mammals, will be critical to a full understanding of the potential and limitations of heart regeneration in humans.

In the heart, cardiac-resident multipotent stem cells are rare in adults and compromised by aging, but retain the capacity to be expanded in vitro and augmented in vivo to stimulate repair.

Understanding the mechanisms that regulate stem and stromal cells functions in this largely post-mitotic organ, and how they communicate with and direct immune cells, vascular cells and cardiomyocytes, will provide the basis for the development of new therapeutic approaches to address the common cardiac conditions affecting large segments of the population.

The objectives of this theme are being met by novel collaborative research projects amongst the laboratories of Theme Leaders and our network of Affiliates, and by coordination of research activities through Theme meetings. Recent work has identified endogenous molecules that influence innate cardiac repair mechanisms such as c-kit, Tbx20, PDGF, thyroid hormone, IGF-1 and Neuregulin 1, and these are informing the design of therapeutic intervention in heart disease.

Analysis of the composition and networks underpinning cardiac fibroblasts and other stromal compartments is now an intense focus of the Theme.

The Rosenthal laboratory has undertaken a detailed FACS-based characterisation of stromal populations, and participates in a new interactive network initiative, CARFMAP, aimed at discovering and collating signalling and genome-wide data relevant to fibroblast biology. They are mining large data sets related to cardiac regeneration and repair, comparing neonatal and adult mouse hearts to other vertebrate models. In the Harvey and Nordon laboratories, single cell analysis of cardiac stem cell behaviours and gene expression is a key platform that is now being extended to analysis of single cell transcriptomes and epigenetic states.

Molecular and genetic analysis of cardiac stem and stromal cells is helping to determine the nature of the quiescent state, chromatin landscapes, memory of organ-of-origin and implications for outputs, including the metabolic basis of stem cell character and organ dialogues. Microfluidic analysis of novel mechanisms of stem cell action through physical transfer is another novel area of collaborative research in the Rosenthal and Cooper-White laboratories.

Defining the role of the immune system and other non-myocyte populations in stem cell maintenance and cardiac repair, including resident marcophages and T-regulatory cells, is also area of intense study under this theme.

The Graham and Harvey laboratories have recently shown how adolescent and mature mammalian cardiomyocytes can be stimulated to divide, overturning the long-held paradigm that these cells terminally withdraw from the cell cycle in neonates. Division can be induced by hormones, growth factor pathways, or after inhibition of a stem cell-associated tyrosine kinase receptor.

"HUMAN HEART REGENERATION IS THE ULTIMATE GOAL OF THESE PARALLEL LINES OF INVESTIGATION."

System biology approaches are now being used to unravel the molecular basis underlying these remarkable shifts in CM proliferative capacity, including epigenetics, non-coding RNA profiling, and the identification of novel molecules that induce cell-cycle re-entry of adult mammalian CMs. Expansion of human pluripotent stem cell-derived CMs in vitro is also being used to model molecular control of the CM cell cycle.

Human heart regeneration is the ultimate goal of these parallel lines of investigation. Effective heart repair after ischemic injury in humans will involve a myriad of cellular and molecular dialogues. These are needed to stabilize the injury site against high hemodynamic pressures, resolve scar and replace it with appropriately vascularised myocardium that is functionally integrated with the rest of the organ and responsive to systemic and hemodynamic control of output on a moment-by-moment basis. These studies of the Cardiac Theme are identifying the key nodes in this process for intervention and augmentation.

Theme: Haematopoiesis

Professor Doug Hilton and **Professor David Haylock**

Stem Cells Converting into Blood Cells

A main aim for this theme is to define parameters for the generation of new sources of Haematopoietic Stem Cells (HSC) by either conversion of human pluripotent stem cells or by direct reprogramming of mature blood cells to HSC.

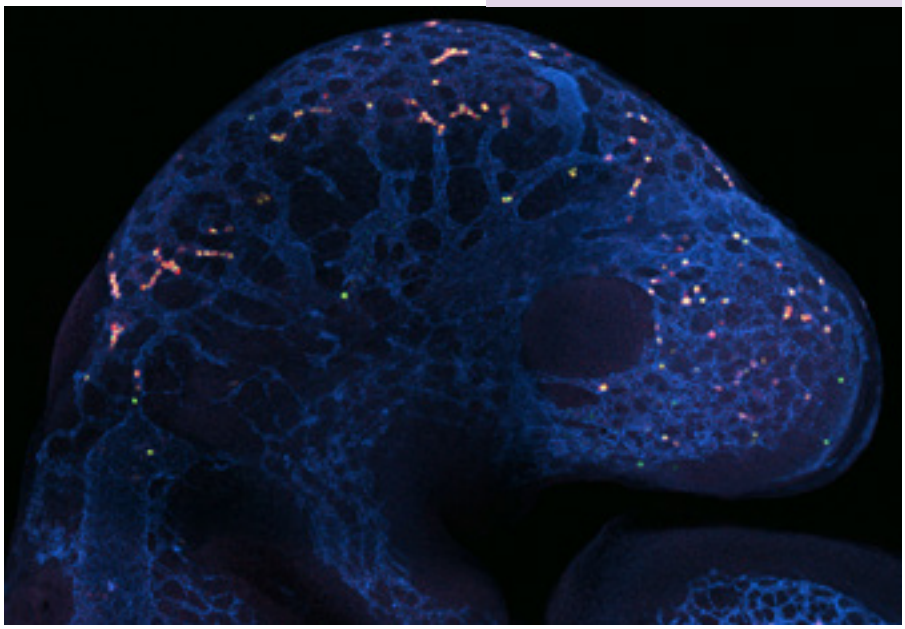
The last year has seen strong progress on all projects. Of note are the outcomes arising from the project lead by Lars Nielsen (UQ) which aims to evaluate the compatibility of their recent protocols for HSC expansion with protocols for expansion and differentiation along the neutrophil lineage and to engineer cells capable of multi-lineage differentiation for use in the manufacture of transfusable blood products. The successful completion of technical and economic feasibility assessments has led to the Centre for Commercialization of Regenerative Medicine, Canada, taking up options on two of the Nielsen group patents.

The project led by Doug Hilton and Samir Taoudi (WEHI) aims to address the mechanisms of early blood fate in the embryo. Hilton and Taoudi have begun to dissect the developmental pathway of early platelet formation in the yolk sac.

Key outcomes to date include:

- the discovery that the first phase of platelet formation is via a primitive, progenitor-independent, pathway;
- that the MPL-THPO signaling axis, which is crucial for megakaryocyte and platelet production in the adult, is not required for platelet production in the early embryo; and,
- that platelet production does not occur via formation of megakaryocyte proplatelet extensions, as in adult thrombogenesis, but rather by a process of direct release from megakaryocytes.

Early platelet-forming cells (red and yellow) within the vasculature (blue) of the early mouse embryo. Courtesy of Kathy Potts and Samir Taoudi (WEHI).



The Hilton and Taoudi team have also made great progress in their investigation of the molecular control of blood stem cell formation in the embryo: they have identified a number of putative stem cell controlling genes from their expression dataset of the developing stem cell lineage in the embryo, and are using CRISPR/Cas9 technology to knock out these target genes in the early embryo. Using this approach, they have identified a novel transcription factor that regulates stem cell numbers during both embryonic development and adult life.

This work is complemented by studies conducted by Andrew Elefanty (UoM) to generate genetically modified pluripotent stem cells for lineage tracing and differentiation studies. They have developed and validated a range of new constitutive and inducible systems via the GAPTrap reporter system, and have identified factors involved in generating human blood. The publications describing these develops are forthcoming.

The project lead by Andrew Laslett (CSIRO) aims to understand the extent to which stem cells derived from reprogramming strategies are able execute the functions of their native counterparts.

The addition of a project overseen by Doug Hilton and Shalin Naik (WEHI) adds cutting- edge single cell technologies to the program. This project aims to use molecular barcoding and single-cell gene expression to develop a high- resolution fate map of haematopoiesis and to identify novel genes involved in the earliest lineage priming events.

Doctoral Graduates

We would like to congratulate the following postgraduate students for completion of their studies in 2015

Harleen Basrai (PhD) supervised by Ann Turnley (UoM). Title of Thesis *Regulation of newborn neuron survival and the inflammatory cell response after traumatic brain injury by Suppressor of Cytokine Signalling-2 (SOCS2).*

Stephanie Bellmaine (PhD) supervised by Martin Pera (UoM). Title of Thesis *Controlling stem cell fate via chemical inhibition of DYRK kinases.*

Bianca Borchin (PhD) supervised by Jose Polo (ARMI, Monash). Title of Thesis *Directed derivation and FACS-mediated purification of PAX3+/PAX7+ skeletal muscle precursors from human pluripotent stem cells.*

Marion Brunck (PhD) supervised by Lars Nielsen (AIBN, UQ). Title of Thesis *RNA CaptureSeq: Targeted Sequencing for Comprehensive Transcriptome Studies*.

Xiaoli Chen (PhD) supervised by Peter Gray (AIBN, UQ). Title of Thesis *Development of a Culture Platform for the Expansion of Pluripotent Human Embryonic Stem Cells with the use of Nanopolymers*.

Duncan Crombie (PhD) supervised by Alice Pebay (UoM). Title of Thesis *Investigating Friedreich ataxia ophthalmopathy and cardiomyopathy using induced pluripotent stem cells*.

Frisca Frisca (PhD) supervised by Alice Pebay (CERA, UoM). Title of Thesis *Lysophosphatidic acid signalling in neurogenesis and the establishment of midline axis.*

Leon Teo (PhD) supervised by James Bourne (ARMI, Monash). Title of Thesis *Development and repair of the nonhuman primate brain: role of the eph and ephrins.*

Lulu Xing (PhD) supervised by Tobias Merson and Trevor Kilpatrick (The Florey). Title of Thesis *Investigation of the relative contribution of neural precursor cells and oligodendrocyte progenitor cells to myelin repair.*

Education, Ethics, Law and Community Awareness Unit

Our Education, Ethics, Law and Community Awareness Unit aims to provide the Australian community with reliable and authoritative information about developments in stem cell science, and in particular the promise of innovative therapies, as well as address the possible ethical and societal impact of such discoveries.

Throughout 2015 SCA has partnered with key national and international research organisations, patient advocacy groups, teacher associations, academics, industry and professional bodies to deliver a suite of educational activities. We have also continued to raise awareness about the policy implications of stem cell research, especially related to the sale of unproven stem cell 'treatments' in Australia and abroad.

Outreach and Communication Activities

Getting reliable information about stem cell research can be challenging for anyone seeking a solution to troubling health problems, or even those simply interested in learning more about this exciting new area of medical research.

In 2015 SCA supported the ISSCR to expand their *Closer Look at Stem Cells* website

www.closerlookatstemcells.org

Launched in April, this website is a comprehensive destination for those interested in stem cell science and research being conducted across the globe. The site encourages the reader to learn more about the science that underlies a given treatment, as well as the principles and practices that should be followed when taking stem cell research from the laboratory to the clinic.

At a local level we collaborated with the National Stem Cell Foundation of Australia to update *The Australian Stem Cell Handbook* - an electronic resource designed to help those interested in stem cell science to ask the right questions when researching stem cell treatments – and also partnered with the ASSCR to develop a multimedia public engagement platform featuring video and the *Stem Cell Essential* public information site.

During 2015, we continued to support high school teachers to bring stem cell science into the classroom.

In May we held an event for over 100 year nine and ten students and their teachers at UQ. Held in conjunction with Cell Reprogramming Australia, the event provided an opportunity for students to meet leading Australian scientists involved in the exciting field of reprogramming

"...THE EVENT WAS INSPIRATIONAL FOR STUDENTS WITH THE CONTENT PITCHED AT A LEVEL EASILY UNDERSTOOD..."

In an effort to take stem cell science out of the laboratory and into the community, we also held a number of public events that provided an opportunity for researchers to talk about their work and interested members of the public to ask questions. Topics included: regenerative medicine; the role of stem cell research in understanding brain development and neurological conditions; and whether eggs and sperm can really be made from stem cells. Coinciding with a visit to Australia by Professor Timothy Caulfield, Canada Research Chair in Health Law and Policy at the University of Alberta, we also held a workshop – *Selling Stem Cells* – to highlight how hype, hope, celebrity culture and marketing converge to imply 'miracle' stem cell treatments are available now. This event attracted an audience of over 150 people. Audio of the event can be accessed via

www.youtube.com/embed/v-hMTEkiKeg.

and pluripotency. As one teacher mentioned, the event was "inspirational" for students with the content pitched at a level easily understood by the students and in a forum where they could "ask any questions they had both on and off the topic of stem cells". To further support teachers, SCA worked with the Genome Technology Access Center and a group of Victorian teachers to develop new resources for teachers and students as part of ReMSTEP, a Victorian initiative to reconceptualise mathematics and science teacher education.

Core to our outreach and communication activities was the SCA website and social media activities. During 2015 we have responded to more than 250 public enquiries, and had over 90,000 visitor to our website where we posted 30 news items about our members and their activities. Our @StemCellAus twitter feed now attracts over 1,800 followers.

StemCells@UQ: Professor Ernst Wolvetang discussing pluripotency with Brisbane high school students. Courtesy of AIBN, UQ.

Research & Policy

Stem cell science remains a cornerstone of medical research. From using stem cells to better understand how we develop, what occurs during disease and injury, to the development of possible new cell-based therapies.

Cutting edge research such as induced pluripotent stem cells – where cells can be created directly from a patient – now means it is possible to screen for new pharmaceuticals or biologics, making the prospect of precision medicine a step closer.

However, for many in the community excitement about the progress and possibilities that stem cell research may offer, coupled with compelling direct-to-consumer advertising, has fostered a 'stem cell' industry where so-called treatments appear to be available now but without any credible scientific evidence to support the marketing claims.

For many years SCA has been an outspoken critic of such practices which effectively by-pass the clinical trials framework.

During 2015, we joined colleagues from around the world to update the ISSCR *Guidelines for Clinical Translation of Stem Cells* and thereby set standards on bring science from the laboratory to the clinic. SCA also provided a submission to the Therapeutic Goods Administration (TGA) in response to their request for feedback on possible ways to close what we consider to be a loophole in the current Australian regulations that govern autologous cell therapies – where the patient's own cells are used.

In addition, recognizing that professional standards are also an important consideration, we have continued to liaise with the Australian Health Practitioner Regulatory Agency (AHPRA) and professional bodies such as the Royal Australasian College of Physicians to enhance professional understanding and awareness about these unproven, and in many cases unfounded, practices.

A key focus of our research program remains capturing the experience of Australians who have sought unproven stem cell treatment here, overseas or have contemplated doing so.

Since 2012 SCA has been part of an international multi-disciplinary research project, *High hopes, high risk? A sociological study of stem cell tourism*. Our work was presented at numerous national and international conferences including the ASSCR conference and the European Association for the Study of Science and Technology.

In September we held a workshop to mark the conclusion of this project where we discussed community expectations in stem cell science and the complex roles of hope, hype and regulatory and ethical oversight. We were delighted to attract representatives from many fields of academia as well as from TGA, AHPRA and numerous patient advocacy groups. The group will publish a book, *Stem Cell Tourism: Hope, Expectations and New Technologies* with Palgrave Macmillan in 2016.

Megan Munsie was also part of an international team awarded an ARC Linkage grant in 2015 to develop an ethical and regulatory framework for the use of autologous therapies in Australia with colleagues at The University of Sydney, Australian National University and the University of Singapore.

Continuing our interest in policy around the use of stem cells in research, we co-authored a *Nature Methods* paper calling for wide consultation about the implications of using pluripotent stem cells to mimic early stages of development.

The capacity to be able to recapitulate what until recently was only possible in vivo opens up the possibility for future health benefits, but also raises ethical and regulatory issues that need to be discussed with the public and policy makers alongside the technical evolution.

The Education, Ethics, Law and Community Awareness Unit is supported by The University of Melbourne and Monash University.

conSCIENCE

Stem Cell Loophole Must Be Closed

Unproven stem cell treatments are being offered in Australia after regulations were loosened.

The potential to use a patient's own stem cells to treat currently intractable conditions has the capacity to revolutionise medicine. However, despite more than a decade of intensive research on a number of stem cell systems, the only proven and approved stem cell therapy available for Australian patients is the transplantation of stem cells from bone marrow to treat diseases of the blood and immune system. Other stem cell applications are only just starting to be evaluated in clinical trials.

Despite this there has already been a proliferation of commercial clinics offering unproven stem cell therapies for a wide range of conditions. These treatments are not cheap – often with an asking price around $10,000 per treatment.

Bizarrely, single preparations of "autologous" (patient-derived) stem cells are being injected to treat diseases as diverse as osteoarthritis and autism, injuries including spinal fracture, cosmetic applications and ageing. Such practices rely on a business model that exploits the hope and hype surrounding stem cells, the expectations of desperately ill and frustrated patients seeking the latest therapies, and the trust normally afforded medical professionals.

... there has already been a proliferation of commercial clinics offering unproven stem cell therapies for a wide range of conditions.

There is real potential to harm patients because the behaviour of stem cells is unknown and unpredictable in many circumstances, particularly where the cells have been manipulated outside of the body. Practitioners involved in selling unproven treatments flaunt a number of medical ethical standards, and often misrepresent the nature of cells harvested, the benefits of therapies, and even their own expertise.

Due to a loophole in our regulations, Australia is set to become a destination for stem cell tourism. In 2011 the Therapeutic Goods Administration (TGA), which is responsible for ensuring that therapeutic goods in Australia are of an acceptable standard, introduced an exemption from undue regulatory interference for routine autologous therapies such as cardiac bypass grafts.

This measure inadvertently created a loophole for opportunistic clinics, because the exemption also effectively allows clinicians to perform any type of unproven cell therapy as long as cells are obtained from the patient and used under the supervision of a medical practitioner for a single indication.

However, there are currently no standards for quality control of the manufacturing process or delivery of the cell preparation, and there is no obligation for practitioners to attest to the safety or efficacy of therapies. There is also no requirement to follow-up on the well-being of patients, or to report adverse events or findings in the scientific literature. The normal process of proving the safety of new cellular therapies through step-wise clinical trials has been completely bypassed.

The opinion of international experts and societies is clear: that provision of unproven autologous therapies to large numbers of individuals for profit is unethical. Local stem cell, patient and medical professional bodies have also spoken out, including the National Stem Cell Foundation of Australia, Motor Neuron Disease Australia, the Australian Rheumatology Association and The Royal Australasian College of Physicians. Even other government agencies such as the National Health and Medical Research Council recognise the dichotomy between the TGA loophole and their duty to promote and support evidence-based medicine.

In January the TGA signalled that it would consider tightening the regulatory framework for autologous stem cell therapies, and sought community feedback on possible solutions. A variety of options were flagged. Under all but the status quo option, the TGA exemption would only apply if autologous cells are minimally manipulated and used in applications where their physiological function remains identical to their role in the tissue from which they are harvested.

Expert scientific bodies including the Australian Academy of Science and Stem Cells Australia have called for the highest level of regulation, whereby cells would need to be registered with the TGA as therapeutic goods, with the risk and benefits assessed for evidence of efficacy, safety and quality control during manufacturing. There would also be an obligation to report adverse findings. None of these requirements would hinder the exploration of novel therapies through the normal channels of clinical trials.

This move towards robust community debate and education provides an opportunity for Australia to arrest the most egregious exploitation of the current regulatory loophole without compromising patient access to novel therapies. Ultimately, evidence and a duty of care to patients must underpin responsible translation of stem cell science and regenerative medicine into accepted clinical practice.

There is too much at stake to ignore due diligence and appropriate regulatory oversight. We eagerly await the TGA's next step.

Richard Harvey, Martin Pera and Megan Munsie are Chief Investigator, Program Leader and Associate Investigator, respectively, for Stem Cells Australia.

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Article reprinted with permission from Australasian Science, June 2015.

Research Support

Welcome to Stemformatics: the collaboration platform of SCA

The stepping stone for bioinformatics and stem cell biologist collaboration

Explore the portal

Based at UQ, Stemformatics is a portal to a series of public experiments describing mouse and human stem cells and how they differentiate to become mature cells, tissues and organs. **We'd like you to explore our free service,** which is meeting an increasing demand from the international community.

We provide a fast way to find and visualise genes in exemplar stem cell datasets; high-quality gene expression and annotation data of interest to the stem cell biologist that has been designed to be quick and easy to use.

You'll find data from leading stem cell laboratories in a format that is easy to search, visualise and export. All of the data on the Stemformatics site has been hand-picked, curated and checked for experimental reproducibility and design quality, and normalised in-house. **Stemformatics provides a much-needed interface between large, and often complex, gene expression datasets and stem cell researchers who lack bioinformatics training.**

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Stemformatics will allow you to answer the following questions:

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Get going today!

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Email: info@stemformatics.org

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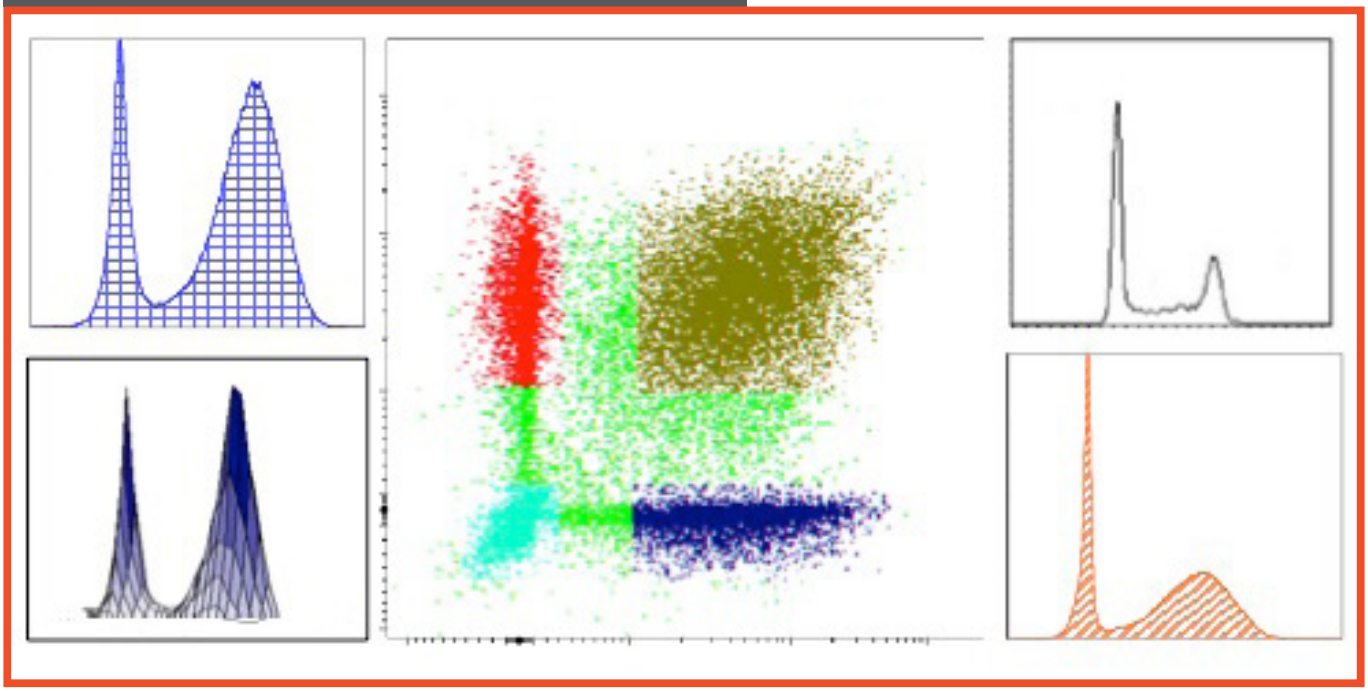

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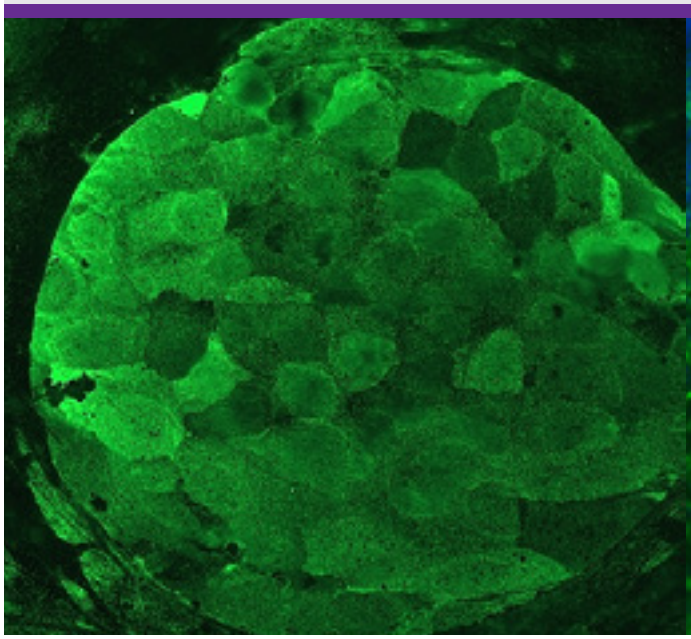
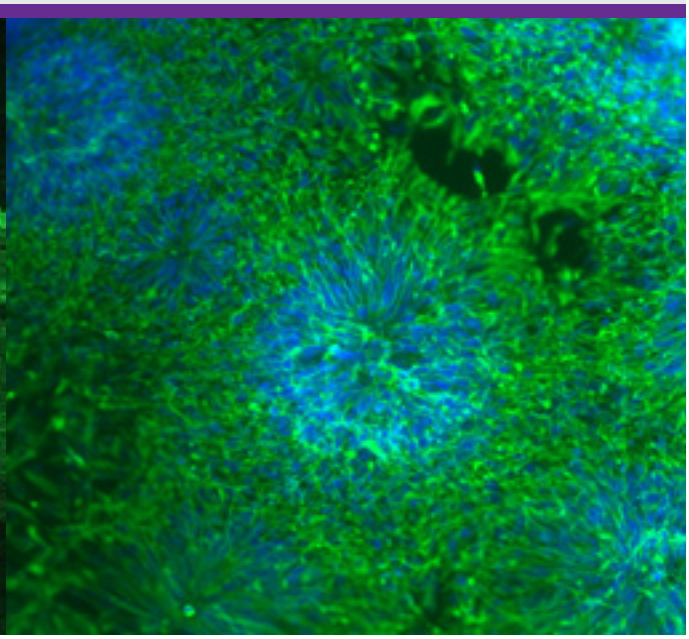




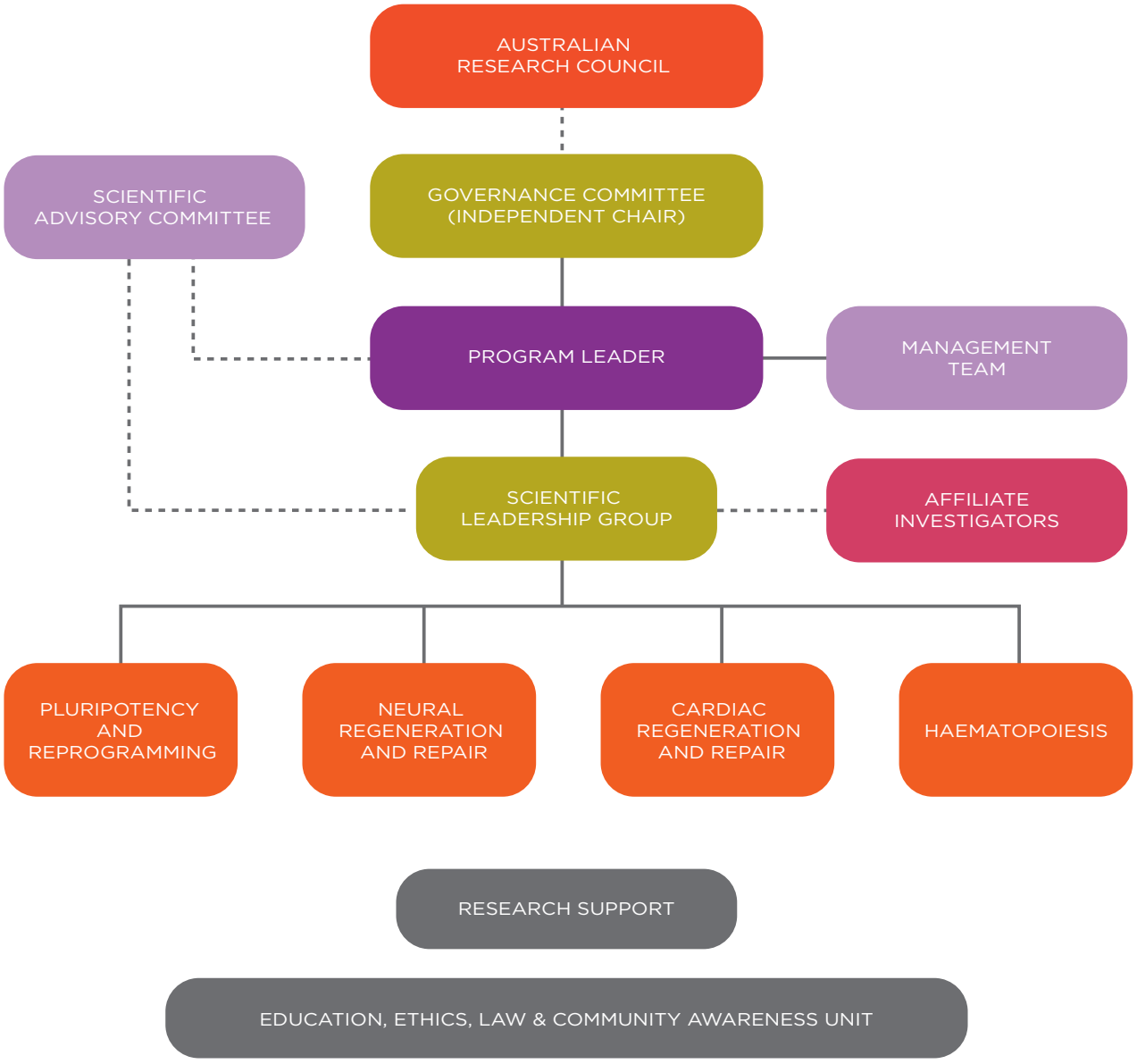
STEMCELLS AUSTRALIA · THE UNIVERSITY OF MELBOURNE · THE UNIVERSITY OF QUEENSLAND AUSTRALIA

Leadership and Governance

Stem Cells Australia operates under an agreement between the Australian Research Council, with the University of Melbourne as the administering organisation, and its partners.

The Initiative is an unincorportated joint venture of The University of Melbourne, Monash University, University of Queensland, University of New South Wales, Walter and Eliza Hall Institute of Medical Research, Victor Chang Cardiac Research Institute*, Florey Institute of Neuroscience and Mental Health and the Commonwealth Scientific and Industrial Research Organisation.

**VCCRI does not participate in hESC research*



Governance Committee

The GC has representatives from each of the partner organisations and an independent Chair. The Committee ensures that the initiative is well managed with a particular focus on endorsement of the research program and the budget. The GC provides strategic advice to the Program Leader. The Research Program and Individual Projects require approval by this Committee. The Committee meets twice per year.

Professor David de Kretser
Independent Chair

Dr Julian Clark
Walter & Eliza Hall Institute of Medical Research (WEHI)

Professor Ross Coppel
Monash University (Monash)

Dr Henry de Aizpurua
The Florey Institute of Neuroscience and Mental Health (The Florey)

Ms Britt Granath
Victor Chang Cardiac Research Institute (VCCRI)

Professor Mark Hargreaves
The University of Melbourne (UoM)

Professor Robyn Ward
University of Queensland (UQ)

Dr Keith McLean
Commonwealth Scientific and Industrial Research Organisation (CSIRO)

Professor Peter Gunning
University of New South Wales (UNSW)

Scientific Advisory Committee

Consisting of internationally renowned national and international experts in the field of stem cell science, the SAC provides strategic advice to the scientific leader on the research direction of the initiative and will provide independent evaluation and feedback on the research performance and science. The committee members meet once a year at the annual retreat.

Professor Christine Mummery
Leiden University Medical Centre, The Netherlands *Head of pluripotent stem cells and differentiation to cardiovascular cells*

Professor and Chair of Developmental Biology at Leiden University Medical Centre, Professor Mummery pioneered studies on heart muscle cells (cardiomyocytes) made from human embryonic stem cells and was among the first to inject them into a mouse heart after a heart attack. Her present interests are focussed on using stem cell derived cardiomyocytes and vascular cells as disease models for drug discovery.

Professor Andras Nagy
Lunenfeld-Tanenbaum Research Institute, Canada *Canadian Research Chair in Stem Cells and Regenerative Medicine*

Canadian Research Chair in stem cells and regenerative medicine, Professor Nagy heads a team of 50 researchers on project Grandiose which studies the process of creating stem cells and have demonstrated advances in stem cell creation which are expected to lead to improved treatments for a number of diseases. In 2005, Professor Nagy was the first to create new stem cell lines in Canada and in 2009, he demonstrated how cells could be changed into stem cells without the introduction of potentially damaging viruses and was included that year in Scientific American's Top 10 Honor Role. Professor Nagy and his research group have discovered a new type of stem cell, called the F-Class iPS cell. Nagy holds an Adjunct Professorship at Monash University.

Professor Hideyuki Okano
Keio University, Japan *Dean of Keio University Graduate School of Medicine*

Professor Okano conducts basic research in the field of stem cells including adult neurogenesis, neural stem cells, neural crest stem cells, and RNA binding proteins and translational research; in particular, the development of cell replacement strategies for injured spinal cord using somatic neural stem cells from induced pluripotent stem (iPS) cells.

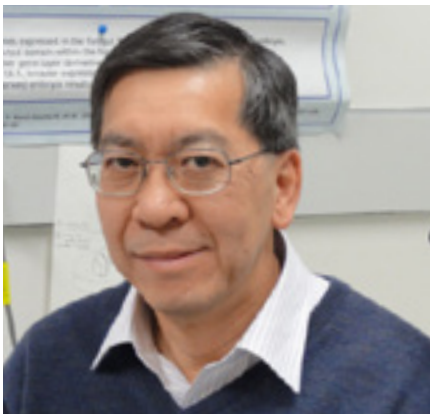
His group first achieved functional recovery of spinal cord injury in model animals including non-human primates by the transplantation of iPS cell-derived neural stem cells and succeeded in generating transgenic non-human primates with germline transmission using common marmoset.

He aims to establish and provide genetically modified non-human primate models for neurodegenerative disorders. He has received several awards and honors including the Medal of Honor with Purple Ribbon in 2009.

Professor Michael Rudnicki
Ottawa Hospital Research Institute, Canada *Director of the Regenerative Medicine Program and the Sprott Centre*

An Officer of the Order of Canada (2013), and the recipient of the prestigious 2014 Till & McCulloch Award for his ongoing work in stem cell and regenerative medicine research, Professor Rudnicki holds the Canada Research Chair in Molecular Genetics and works to understand the molecular mechanisms that regulate the determination, proliferation, and differentiation of stem cells during embryonic development and during tissue regeneration.

The lab has conducted leading studies into both embryonic myogenesis and the function of muscle stem cells in adult regenerative myogenesis, in particular to understand the molecular mechanisms that regulate the function of satellite cells in skeletal muscle. They identified Pax7 as a transcription factor required for the specification of satellite cells, and identified Wnt7a signaling as playing an important role in muscle stem cell function.



Professor Patrick Tam
Childrens Medical Research Institute *Deputy Director, NSW Australia*

The Scientific Advisory Committee Chair, Professor Tam's research focuses on the cellular and molecular mechanisms of body patterning during mouse development and the biology of stem cells. He is internationally recognised for pioneering the application of micromanipulation and embryo culture, for analysing the development of mouse embryos, and fate mapping of the mouse germ layers and embryonic gut. His other current research also covers the genetic models of X-linked diseases and the molecular controls of eye development. In recognition of his research achievement, Professor Tam was awarded the President's Medal of the Australia and New Zealand Society of Cell and Developmental Biology in 2007.

Professor Peter Zandstra
University of Toronto, Canada
Canadian Research Chair in Stem Cell Bioengineering

Professor Zandstra's research – motivated by the hypothesis that the appropriate engineering of the cellular microenvironment will enable robust and efficient manipulation of stem cell self-renewal and differentiation – is focused on understanding the interface between microenvironmental control and the endogenous and intracellular networks that underlie stem cell fate decisions.

Areas of work include quantitative spatial and temporal control of embryonic stem cell self-renewal, bioprocesses for the generation of blood and cardiac cells from embryonic stem cells, and control of intercellular signaling networks to grow human blood stem cells. Ultimately, the goal is to enable stem cell based therapies and technologies to be developed for health and welfare.

Our People

Scientific Leadership Group

Chaired by the Program Leader, the Scientific Leadership Group is a group of senior scientists consisting of theme leaders of each of the four themes. This core group is the scientific powerhouse of SCA and are responsible for the delivery of the scientific research programs. They are the regular referral point for the Scientific Leader and assist in the development of the annual research work plan and budget for the initiative.

Meeting bi-monthly, the Scientific Leadership Group assist the Scientific Leader in monitoring the operations and activities of the initiative and are the liaison and co-ordination points for the activities of the initiative within the broader SCA community.

Professor Martin Pera
Program Leader UoM, WEHI, The Florey

Professor Perry Bartlett
Queensland Brain Institute (QBI) UQ

Professor Peter Gray
Australian Institute for Bioengineering and Nanotechnology (AIBN), UQ

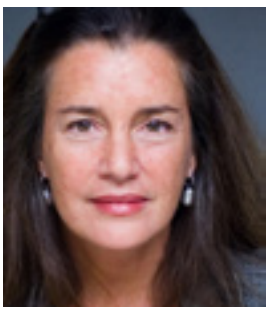
Professor Richard Harvey
UNSW, VCCRI

Professor David Haylock
CSIRO

Professor Doug Hilton
UoM, WEHI

Professor Trevor Kilpatrick
UoM, The Florey

Professor Melissa Little
Institute of Molecular Bioscience (IMB) UQ



Professor Nadia Rosenthal
Australian Regenerative Medicine Institute (ARMI), Monash

Chief and Partner Investigators

The Chief and Partner Investigators are the senior researchers of the initiative and the project leaders of SCA funded projects.

Professor Warren Alexander
Partner Investigator
WEHI

Professor Perry Bartlett
Chief Investigator
QBI, UQ

Associate Professor James Bourne
Chief Investigator
ARMI, Monash

Professor Justin Cooper-White
Chief Investigator
AIBN, UQ

Professor David Gardner
Chief Investigator
UoM

Professor Robert Graham AO
Chief Investigator
UNSW, VCCRI

Professor Peter Gray
Chief Investigator
AIBN, UQ

Professor Richard Harvey
Chief Investigator
UNSW, VCCRI

Professor David Haylock
Partner Investigator
CSIRO

Professor Doug Hilton
Chief Investigator,
UoM, WEHI

Dr Robin Hobbs
Chief Investigator
ARMI, Monash

Professor Trevor Kilpatrick
Chief Investigator
UoM, The Florey

Associate Professor Andrew Laslett
Partner Investigator,
CSIRO

Professor Melissa Little
Chief Investigator, IMB,
UQ

Professor Lars Nielsen
Chief Investigator,
AIBN, UQ

Associate Professor Susie Nilsson
Partner Investigator,
CSIRO

Dr Nathan Palpant
Chief Investigator
IMB, UQ

Professor Martin Pera
Chief Investigator
UoM

Associate Professor Jose Polo
Chief Investigator
ARMI, Monash

Professor Nadia Rosenthal
Chief Investigator,
ARMI, Monash

Associate Professor Christine Wells
Chief Investigator,
AIBN, UQ

Professor Brandon Wainwright
Chief Investigator,
IMB, UQ

Associate Professor Ernst Wolvetang
Chief Investigator,
AIBN, UQ

Associate Investigators

These roles are generally involved in SCA funded projects through a Chief Investigator, and are not usually directly supported financially.

Professor Robert Capon
UQ

Dr Mirella Dottori
UoM

Professor Andrew Elefanty
Murdoch Childrens Research Institute (MCRI)

Dr David Elliott
MCRI

Dr Tobias Merson
The Florey

Professor Michael Monteiro
AIBN, UQ

Associate Professor Megan Munsie
UoM

Professor Robert Nordon
UNSW

Dr Clare Parish
The Florey

Associate Professor Alice Pébay
CERA, UoM

Dr Joy Rathjen
UoM

Professor Pankaj Sah
UQ

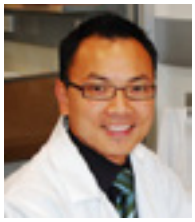
Professor Ed Stanley
MCRI

Dr Lachlan Thomson
The Florey

Associate Professor Ann Turnley
UoM

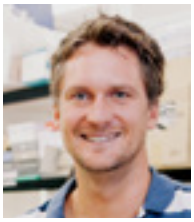
Affiliate Investigators

Stem Cells Australia's Affiliate Investigators are leading Australian and International stem cell researchers from outside our direct network whose vision and leadership further strengthen our initiative.



Dr James Chong
University of Sydney

Professor Peter Currie
ARMI, Monash



Dr James Hudson
UQ

Dr Kazu Kikuchi
VCCRI

Dr Jason Kovacic
Mount Sinai Hospital and the Cardiovascular Research Centre, New York

Professor Ryan Lister
University of Western Australia (UWA)

Dr Shalin Naik
WEHI

Dr Enzo Porrello
UQ

Dr Mirana Ramialison
ARMI, Monash

Professor Jane Visvader
WEHI

Management Team

The University of Melbourne is the Administering Organisation for the initiative and hosts the core management team. The Management Team is responsible for all aspects of reporting, administration, finance, committee meetings, events and workshops, and communication activities of the initiative.

Professor Martin Pera
Program Leader

Responsible for the overall direction and operation of the initiative. This role encompasses research leadership, management and communication, liaison and development responsibilities

Ms Barbara Power
Business Manager

Provides administrative leadership across the eight nodes of the initiative whilst managing all the financial dealings including grant and contract management, compliance and program management. Also the committee secretary responsible for all the committee meetings.

Associate Professor Megan Munsie
Head - Education, Ethics, Law & Community Awareness Unit

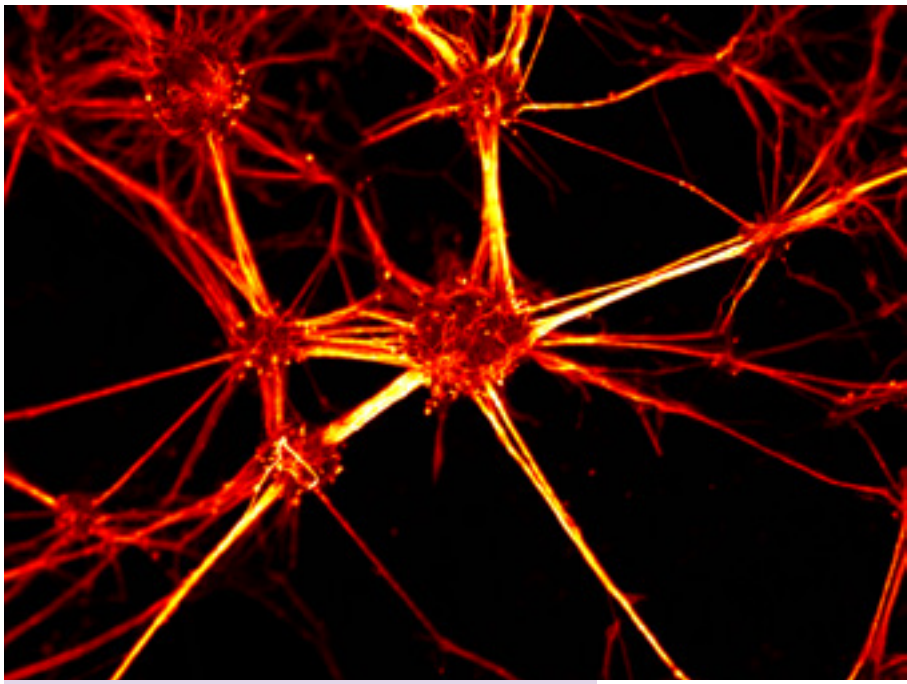
Responsible for SCA's communication, outreach and policy activities, and leads research into ethical and societal issues associated with stem cell science and its clinical applications.

Ms Jennifer Kendall
Executive Assistant to Program Leader

Provides executive administrative support to the program leader. Responsible for office management, HR and student coordination and assistance with event management, marketing and communication activities.

Dr Sandani Udabage
Assistant to Business Manager

Provides general administrative support including; annual retreat logistics, preparation of the annual report and KPI data collection.



Neural Cosmos. Courtesy of Jarmon Lees (UoM).

Platform Technologies Team

Stemformatics Group

Based at UQ, Stemformatics is a collaboration between the stem cell and bioinformatics communities. It provides the Australian stem cell community with a collaborative platform that enables the interrogation of stem cell datasets without formal bioinformatics training.

Associate Professor Christine Wells
Project Leader
UQ

Mr Rowland Mosbergen
Developer
UQ

Mr Othmar Korn
Bioinformatician
UQ

Facilities Teams

1. Flow Cytometry Facility

Based at the Melbourne Brain Centre at UoM, the Flow Cytometry Facility is a purpose-built core facility in Flow Cytometry for use by interested researchers.

Dr Vanta Jameson
Manager
UoM

Mr Joshua Kie
Operator
UoM

2. Stem Cell Core Facilities

Stem Cell Core facilities are based at the Melbourne Brain Centre at UoM and AIBN at UQ. The cores' services are available to the scientists of the two universities as well as other institutions.

Dr Anna Michalska
StemCore Manager
UoM

Ms Katherine Lim
Technical Assistant
UoM

Dr Nilay Thakar
Stem Cells Ltd Manager
UQ

Ms Set Yen Soo
Senior Research Officer
UQ

Ms Nishta Ramnoruth
Senior Research Officer
UQ

Early Career Researchers and Students

Invited for SCA annual retreat, post-doctoral researchers, research assistants and students listed in this report are either directly working in our core stem cells projects or active team members working on other stem cells projects of our senior investigators, thereby part of our broader stem cell network.

Post-Doctoral Researchers

Dr Christelle Adolphe
IMB, UQ

Dr David Anderson
MCRI

Dr Naisana Asli
VCCRI

Dr Poornima Balaji
VCCRI

Dr Daniel Blackmore
QBI, UQ

Dr Alexis Bosman
VCCRI

Dr Nicole Bye
UoM

Dr Ben Cao
CSIRO

Dr Huimin Cao
CSIRO

Dr Ai-Leen Chan
ARMI, Monash

Dr Jarny Choi
WEHI

Dr Giovanna Marisa D'Abaco
UoM

Dr Mauro da Costa
ARMI, Monash

Dr Kathryn Davidson
ARMI, Monash

Dr Carolyn de Graaf
WEHI

Dr Gonzalo Del Monte Nieto
VCCRI

Dr Alison Farley
UoM

Dr Jane Fitzpatrick
AIBN, UQ

Dr Elvira Forte
VCCRI

Dr Milena Furtado
ARMI, Monash

Dr Laura Genovesi
IMB, UQ

Dr Nick Glass
AIBN, UQ

Dr James Godwin
ARMI, Monash

Dr Kylie Greig
WEHI

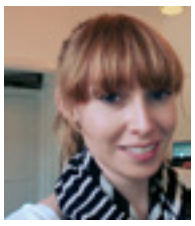
Dr Lorna Hale
MCRI

Dr Linda Harkness
AIBN, UQ

Dr Alexandra Harvey
UoM

Dr Chad Heazlewood
CSIRO

Dr Shen Heazlewood
CSIRO



Dr Jennifer Hollands
The Florey

Dr Melissa Holmes
ARMI, Monash

Dr Jihane Homman-Ludiye
ARMI, Monash

Dr Lieven Huang
ARMI, Monash

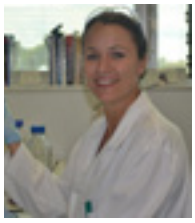
Dr Sandy Hung
UoM

Dr Siiri Iismaa
VCCRI

Dr Vaibhao Janbandhu
VCCRI

Dr Andrew Jarratt
WEHI

Dr Dhanisha Jhaveri
QBI, UQ



Dr Anja Knaupp
ARMI, Monash

Dr Alex Koon
IMB, UQ

Dr Kevin Lau
UoM

Dr Julien Legrand
ARMI, Monash

Dr Ming Li
VCCRI

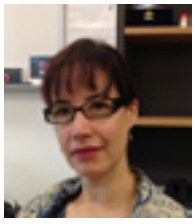
Dr Sue Lim
ARMI, Monash

Dr Juho Makela
ARMI, Monash

Dr Denise Miles
WEHI

Dr Stan Mitew
UoM

Dr Christian Nefzger
ARMI, Monash



Dr Elizabeth Ng
MCRI

Dr Jonathan Niclis
The Florey

Dr Hieu Nim
ARMI, Monash

Dr Carmel O'Brien
CSIRO

Dr Dmitry Ovchinnikov
AIBN, UQ

Dr Alex Pinto
ARMI, Monash

Dr Florian Rohart
AIBN, UQ

Dr Fernando Rossello
ARMI, Monash

Dr Ilaria Stefani
AIBN, UQ

Dr Minoru Takasato
IMB, UQ

Dr Samir Taoudi
WEHI

Dr Jana Vukovic
QBI, UQ

Dr George Wang
UoM

Dr Lina Wang
ARMI, Monash

Dr Raymond Wong
UoM

Dr Katharina Wystub-Lis
VCCRI

Dr Munira Xaymardan
VCCRI

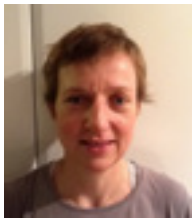
Dr Daniela Zalcenstein
WEHI

Research Assistants

Ms Casey Ah-Cann
WEHI

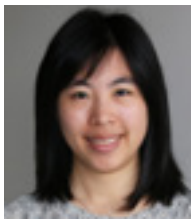
Ms Stacey Andersen
AIBN, UQ

Ms Lisa Azzola
MCRI



Ms Penelope Buntine
AIBN, UQ

Ms Kellie Cartledge
CSIRO



Ms Andrea Chan
VCCRI

Mr Joseph Chen
ARMI, Monash

Mr Han Chiu
IMB, UQ

Mr Hun Chy
CSIRO

Ms Claire Cuddy
UoM

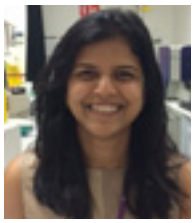
Mr Mitchell de Souza
ARMI, Monash

Ms Pei Er
IMB, UQ

Mr Clayton Friedman
IMB, UQ

Ms Adrienne Hilton
WEHI

Ms Sara Holman
VCCRI



Ms Tejal Kulkarni
UoM

Ms Mai La
ARMI, Monash

Ms Jane Sun
AIBN, UQ

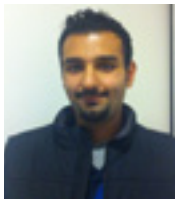
Mr Qi Zhou
CSIRO

Students

Ms Dhanushi Abeygunawardena
VCCRI

Ms Sara Alei
ARMI, Monash

Mr Walaá Alsanie
The Florey



Mr Abdullah Alshawaf
UoM

Ms Deevina Arasartnam
MCRI

Ms Anushree Balachandran
AIBN, UQ

Ms Harleen Basrai
UoM

Ms Stephanie Bellmaine
UoM

Ms Bianca Borchin
ARMI, Monash

Ms Jane Brophy
Monash

Ms Freya Bruveris
MCRI

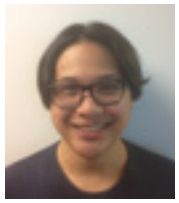
Ms Marion Brunck
AIBN, UQ

Ms Rachael Chatterton (Honours)
CSIRO

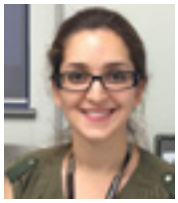
Ms Xaoli Chen
AIBN, UQ

Mr James Cornwell
VCCRI

Mr Duncan Crombie
UoM



Mr Ryan Debuque
ARMI, Monash



Ms Nona Farbehi
UNSW

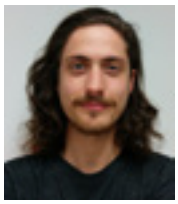
Mr Jaber Firas
ARMI, Monash

Ms Hananeh Fonoudi
VCCRI

Mr Siavash Foroughi
UoM

Mr Patrick Fortuna
AIBN, UQ

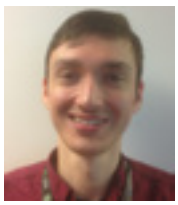
Ms Frisca Frisca
UoM



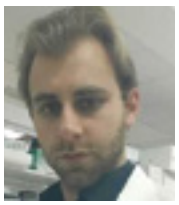
Mr Carlos Gantner
The Florey

Ms Katherine Gill
UoM

Ms Gency Gunasingh
AIBN, UQ



Mr Alexei Ilinkykh
ARMI, Monash



Mr Brett Kagan
The Florey

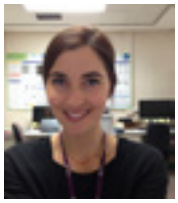
Mr Tim Kao
MCRI

Mr Jack Lambshead
CSIRO

Dr Marcelo Leal
UoM

Mr Jarmon Lees
UoM

Ms Jing Jing (Jane) Li
UNSW



Ms Grace Lidgerwood
UoM

Mr Xiaodong (Ethan) Liu
ARMI, Monash

Mr Riley McMahon (MSc)
UoM

Ms Barbara Maier
IMB, UQ

Ms Elizabeth Mason
AIBN, UQ

Mr Nicholas Matigian
AIBN, UQ

Ms Cristiana Mattei
UoM

Mr Ali Motazedian
MCRI

Ms Amy Nicks
VCCRI

Mr Harish Padmanabhan
AIBN, UQ

Mr Dean Phelan
MCRI

Ms Kathy Potts
WEHI

Ms Elizabeth Qian
MCRI

Ms Terri-Ann Scanlon
UoM

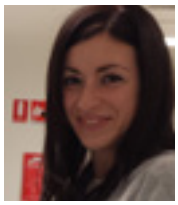
Ms Jessica Schwaber
AIBN, UQ

Mr Rhys Skelton
MCRI

Mr Marcos Soto
AIBN, UQ

Ms Gemma Tan (MSc)
MCRI

Mr Leon Teo
ARMI, Monash



Ms Serena Viventi
UoM

Ms Liyuan Wang
UNSW

Ms Li-Yen Wong
AIBN, UQ

Ms Lulu Xing
UoM

**All students enrolled in PhD unless otherwise stated.*

Performance Tables

Key Result Area 1: Research Performance	2011 KPI		2012 KPI		2013 KPI		2014 KPI		2015 KPI	
	Target	**Actual**	**Target**	**Actual**	**Target**	**Actual**	**Target**	**Actual**	**Target**	**Actual**
Innovative, internationally, competitive research strategically focussed on fundamental stem cell science										
Number of research outputs: Journal Publications	15	29	70	102	80	116	90	141	90	113
Number of Conference proceedings	4	3	20	8	20	9	30	1	30	1
Quality of research outputs										
50% of publications will be in peer reviewed, international journals with an Impact Factor >5	50%	50% (14)	50%	54% (55)	50%	45% (46)	50%	43% (50)	50%	61% (61)
15% of publications will be in journals with Impact Factor >10.	15%	20% (6)	15%	12% (12)	15%	17% (17)	15%	20% (23)	15%	20% (20)
Number of invited talks/ papers/keynote lectures given at major international meetings	3	16	15	46	15	28	15	97	20	67
Patent applications lodged	0	0	0	1*	2	2*	2	0	2	1

Key Result Area 2: Research Training and Capacity Building	2011 KPI		2012 KPI		2013 KPI		2014 KPI		2015 KPI	
	Target	**Actual**	**Target**	**Actual**	**Target**	**Actual**	**Target**	**Actual**	**Target**	**Actual**
Number of postgraduate students working on core SRI research and supervised by SRI members										
Annual	8	14	10	8	9	20	26	18	8	8
Cumulative	8	14	18	22	27	42	53	60	61	68
Number of postdoctoral researchers appointed to the SRI working on core SRI research										
Annual	9	11	20	40	20	9	20	10	20	14
Cumulative	9	11	29	51	49	60	69	70	89	84
Number of postgraduate completions by students working on core SRI research and supervised by SRI members										
Annual	0	0	4	2	5	2	8	7	7	9
Cumulative	0	0	4	2	9	4	17	11	24	20
Qualitative measures of capacity building										
Number of Competitive postdoctoral Fellowships awarded	0	1	1	3	2	4	3	5	2	8
Other awards, short term fellowships, recognitions, appointments, promotions	0	1	9	10	11	17	9	7	10	11

Key Result Area 3: International, National Links and Networks	2011 KPI		2012 KPI		2013 KPI		2014 KPI		2015 KPI	
	Target	**Actual**	**Target**	**Actual**	**Target**	**Actual**	**Target**	**Actual**	**Target**	**Actual**
International Collaboration										
Researchers, fellows attend and present at international conferences	4	16	30	39	35	48	35	28	35	59
Students attending international research conferences	1	1	13	2	14	4	19	22	23	7
Research collaborations with international centres	2	10	3	22	5	16	5	50	5	47
International research funding received	0	$470K	$500K	$1.5M	$750K	$1.2M	$750K	$325K	$750K	$1.2M
National Collaboration Cross-institutional collaboration defined as across research institutions (i.e. collaborating and partner organisations) within SCA										
Annual retreat attended by researchers, fellows, students	N/A	N/A	80%	85%	80%	81%	80%	91%	80%	91%
% publications including cross-institutional authorship	N/A	18% (5)	50%	12%	60%	3%	65%	60%	65%	73%
Number of international visitors and visiting fellows funded with SCA funds staying between 1-2 months (approx)	0	0	2	2	2	4*	2	5	2	3
Number of workshops held/organised by Stem Cells Australia										
National	1	1	1	4	1	7	1	8	1	5
International	0	0	1	1	0	1	1	1	0	1

Key Result Area 4: Knowledge Transfer, Outreach and Communication	2011 KPI		2012 KPI		2013 KPI		2014 KPI		2015 KPI	
	Target	**Actual**	**Target**	**Actual**	**Target**	**Actual**	**Target**	**Actual**	**Target**	**Actual**
Number and nature of commentaries about the Stem Cells Australia's achievements										
Media releases	2	3	5	4	5	6	5	5	6	6
Articles	1	7	3	21	3	20+	4	35+	4	35+
Number of government, industry and business community briefings	1	1	4	7	4	6+	4	4+	4	4
Number and nature of public awareness programs										
Provide tailored resources to community and professional organisations	2	2	4	6	4	11	4	10	4	5
Members participating in community or patient advocacy meetings	3	3	5	16	5	12	10	15	10	13
Engagements with science teachers' associations	1	1	3	2	3	3	3	3	3	4
Website										
Currency of information on Stem Cells Australia's website (number of news items posted)	Website launched Nov 2011			27		51 (+ new sections)		37		30
Number of website hits	2,000	2,559	15,000	22,207	20,000	53,038	20,000	99,570	20,000	90,205

Grants

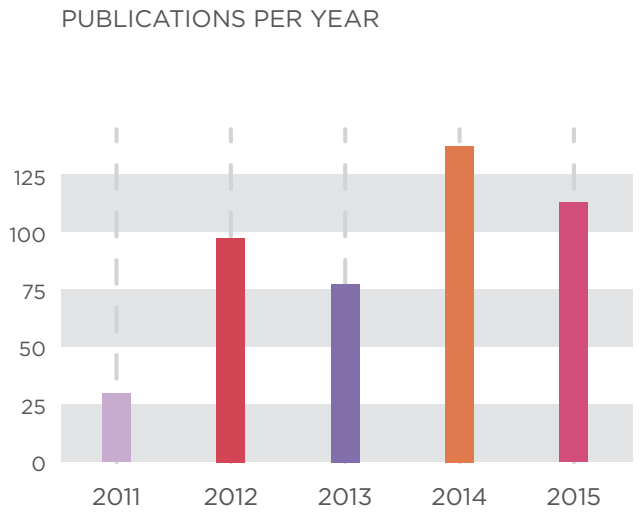
List of grants secured by SCA researchers in 2015.

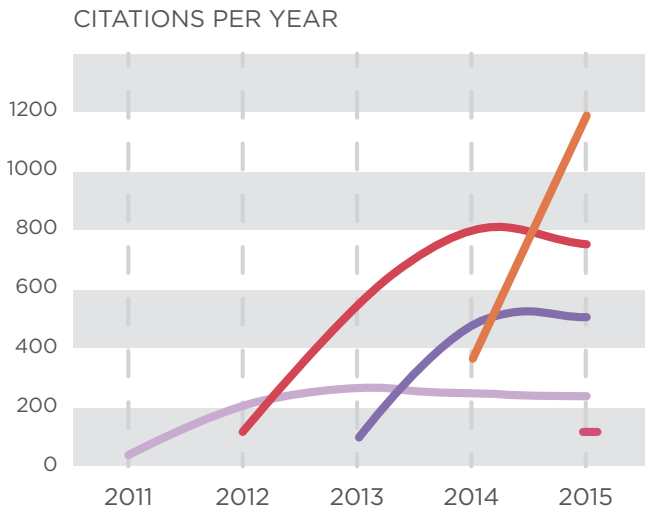
ARC and CRC	$ for 2015	Total value
Megan Munsie et al.: ARC Linkage - "Regulating Autologous Stem Cell Therapies in Australia" ($ 450,000, 2015-18)	450,000	112,500
Lars Nielsen et al.: ARC Linkage Grant - "Improving clostridial toxoid production through molecular fermentation maps" ($788,000, 2015-18)	788,000	197,000
NHRMC	**$ for 2015**	**Total value**
James Bourne: NHMRC Projecr Grant - "A novel treatment for ischemic stroke: preclinical assessment in the nonhuman primate" ($739,154, 2015-17)	739,154	246,385
Andrew Elefanty: NHMRC International Collaborations Grant (CIRM - Tools and Technologies Stem Cells Research) - "A suite of engineered human pluripotent stem cell lines to facilitate the generation of hematopoietic stem cells" ($881,221, 2015-17)	881,221	293,740
Richard Harvey, Robert Graham et al.: NHMRC Program Grant - "Molecular Mechanisms and therapeutic Approaches to Cardiac Development, Regeneration and Disease" ($10,621,535, 2015-19)	10,621,535	2,124,307
Sussi Nilsson: NHMRC Project Grant - "Bone marrow Endothelial Stem Cells have the capacity to form both the endothelial and haemopoietic hierarchies" ($1,394,125, 2015-19)	1,394,125	278,825
Clare Parish: NHMRC Project Grant - "The role of meninges in midbrain dopamine development" ($366,398, 2015-2017)	366,398	122,133
Jose Polo, Andrew Laslett and Mirana Ramialison: NHRMC Project Grant - "Inducing and controlling cellular plasticity" ($763,136, 2015 - 2017)	763,136	254,379
Enzo Porrello: NHMRC Project Grant - "Regulation of endogenous heart regeneration by an anti-fibrotic microRNA" ($427,077, 2015-17)	427,077	142,359
Enzo Porrello: NHMRC Project Grant - "Regulation of heart development and regeneration by DNA methylation" ($535,616, 2015-17)	535,616	178,539
Lachlan Thompson: NHMRC Project Grant - "Pre-Clinical Studies Towards Cell-Based Approaches for Cortical Repair" ($718,472, 2015-17)	718,472	239,491
Jane Visvader: NHMRC Project Grant - "Determination of the cellular origins of breast cancer" ($683,475, 2015-17)	683,475	227,825
Melissa Little: NHMRC Project Grant - "Understanding nephron endowment" ($711,000, 2015-2017)	711,000	237,000
National and International	**$ for 2015**	**Total value**
Mirella Dottori & Lachlan Thompson: Friedreich Ataxia Research Association (USA and Australasia) grant, (US$270,000, 2015-2016)	392,000	196,000
Richard Harvey et al.: NSW Government Office for Health and Medical Research Genomics Collaborative Grant Program, "Discovering the genetic causes of inherited heart disease in babies" ($370,000, 2015)	370,000	370,000
Richard Harvey, Robert Graham, James Chong, Munira Xaymardan et al.: New South Wales Cardiovascular Research Network Research Development Project Grant, "Activation and Rejuvenation of Endogenous Cardiac Stem Cells" ($200,000, 2015)	200,000	200,000
Trevor Kilpatrick: MS Research Australia and Charity Works for MS Incubator Grant , "The role of the receptor tyrosine kinase Tyro3 in central myelination" ($16,000, 2015)	16,000	16,000
Melissa Little: NIDDK Rebuilding the Kidney UH2 award, US$640,000, 2015-17)	928,000	309,333
Alice Pebay et al: Australian Mitochondrial Disease Foundation, incubator grant, "Modeling Leber's Hereditary Optic Neuropathy using human induced pluripotent stem cells" ($25,000, 2015)	25,000	25,000
Lars Nielsen: The Novo Nordisk Foundation Laureate Research Grant, "Explaining the Warburg effect through a detailed kinetic and regulatory model of central carbon metabolism in cultured mammalian cells, (DKK40,000,000 = ~$8 million, 2015-2022)	8,000,000	1,142,857
Alice Pebay et al: Clifford Craig Medical Research Trust Research Grant, "Towards a patient-specific stem cell model of the blinding eye disease glaucoma" ($78,100, 2015)	78,100	78,100
Alice Pebay et al: Retina Australia, "Correcting inherited retina; disease through gene editing" ($39,551, 2015)	39,551	39,551
Enzo Porrello: UQ Major Equipment and Infrastructure Grant - "A state-of-the-art spinning disc confocal microscope" ($335,000, 2015)	335,000	335,000
Ann Turnley: MS Research Australia - "Enhancing myelin repair by attenuating reactive astrocytes" ($180,000, 2015-16)	180,000	90,000
Total	**29,642,860**	**7,456,323**

Publications

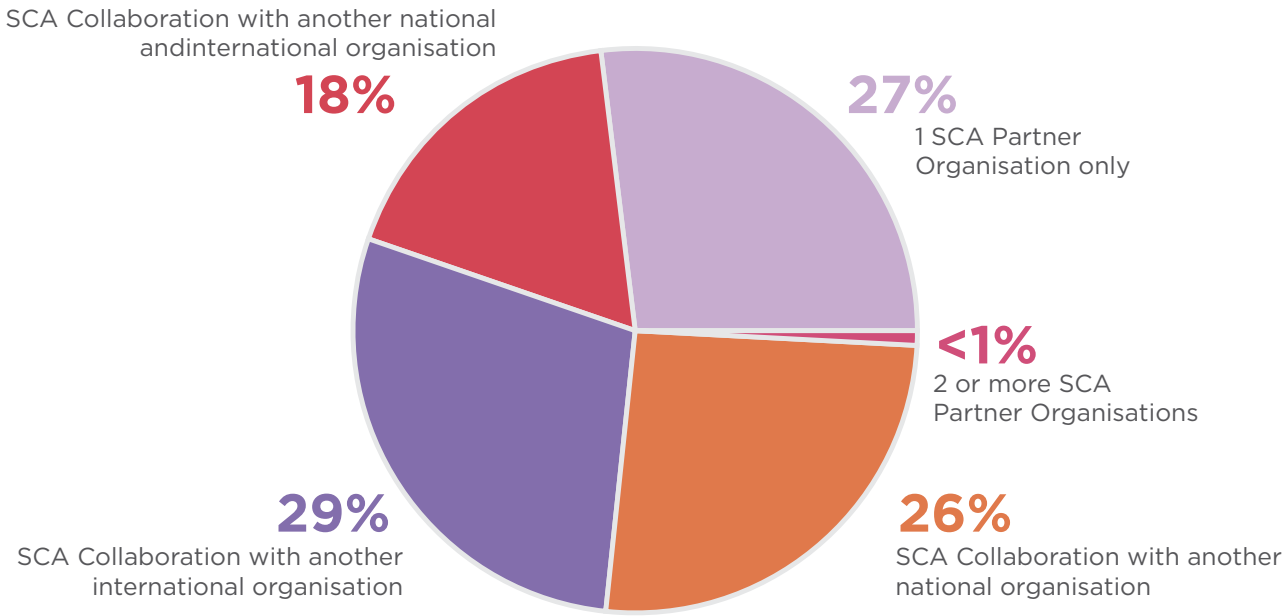
Overview

SCA publications and citation count of published articles.





Collaborative distribution of 2015 journal publications



Most Cited Journal Articles of 2014

Our previous year's work is highly cited. Journal articles authored by SCA Researchers in 2014 were cited over 1,500 times since publication. Names in bold are Chief, Partner, Associate or Affiliate Investigators of SCA. Papers cited more than 10 times as of December 2015..

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2015 Journal Articles with Impact Factor greater than10

Note: researchers in bold are Chief, Partner, Associate or Affiliate Investigators of SCA.

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Conference and Meeting Participation

Invited Speakers

Bartlett P: Invited Speaker *Activation of different neurogenic precursor populations in the hippocampus: Potential for restoring function in disease like dementia,* 6th FAONS Congress and 11th Biennial Conference of the Chinese Neuroscience Society, Wuzhen, Zhejiang, China, 2015.

Bartlett P: Invited Speaker *Regulation and function of neurogenesis in the adult hippocampus,* South University of Science and Technology of China, Shenzhen, China, 2015.

Bartlett P: Invited Speaker *Regulating neurogenic precursors in the hippocampus may reverse cognitive impairment in aged animals*, Australia - China Symposium on Neuroscience, Werribee, Australia, 2015.

Dottori M: Invited Speaker *Modelling the nervous system in a dish using human pluripotent stem cells,* Alzheimer's and Parkinson's Disease Annual Meeting, Brisbane, Australia.

Elefanty A: Invited Speaker HEMO 2015, Brazilian Congress of Hematology, Hemotherapy, and Cell Therapy, Sao Paulo, Brazil, 2105.

Gardner DK: Keynote Speaker *Biomarkers of viability*, IVI International Congress of IVF, Alicante, Spain.

Gardner DK: Keynote Speaker *Laboratory on a chip in ART*, Turkish Society of Clinical Embryology, Cyprus.

Gardner DK: Keynote Speaker *Diagnosis of embryo viability*, Taiwanese Society for Reproductive Medicine, Taiwan.

Gardner DK: Invited Speaker *Metabolism: So much more than ATP*, Oxford University, Oxford, UK.

Gardner DK: Keynote Speaker *Embryo culture and selection*, Japanese Society of Assisted Reproduction, Japan.

Graham RM: Invited Speaker Workshop on Metabolic Profiling, Sanford Institute of Regenerative Medicine, San Diego, USA, 2015.

Graham RM: Invited Speaker *The postnatal regenerative capacity of the mammalian heart*, Cardiovascular Research Institute, Rutgers-New Jersey Medical School, Newark, USA, 2015.

Grey P: Invited Speaker *pNIPAM system*, Scale-up and Manufacturing of Cell-based Therapies IV conference (Engineering Conferences International), San Diego CA, USA, 2015.

Harvey RP: Plenary Speaker *Endocardial plasticity coupled to dynamic ECM flux in cardiac chamber development*, Asia-Pacific Developmental Biology Conference, Xi'an, China, 2015.

Harvey RP: Lecture of Excellence *Molecular Mechanisms of Heart Development and Congenital Heart Disease*, Shanghai Institutes for Biological Sciences, Chinese Academy of Sciences, Shanghai, China, 2015.

Harvey RP: Plenary Speaker *Niche regulation of MSC-like Stem Cells in the Adult Mouse Heart*, Ascona International Workshop on Cardiomyocyte Biology –Integration of Developmental and Environmental Cues in the Heart, Ascona, Switzerland, 2015.

Harvey RP: Keynote Lecture *Trabeculation and Valve Formation During Cardiac Development*, The EMT International Association VIIth meeting, Melbourne, Australia, 2015.

Hobbs R: Invited Speaker *Molecular mechanisms defining germline stem cell heterogeneity and fate*, Gordon conference germinal stem cell biology, Hong Kong, 2015

Little M: Invited Speaker World Congress of Nephrology, Capetown, South Africa, 2015.

Little M: Invited Speaker *Rebuilding the kidney using stem cells*, Society for Development Biology, Snowbird, Utah, July 2015

Little M: Invited Speaker *The directed differentiation from pluripotent cells to kidney cells*, International Workshop for Developmental Nephrology, Utah, July 2015

Little M: Invited Speaker *Recreating the kidney from stem cells: Applications in regenerative and personalised medicine*, COMBIO, Melbourne, September 2015

Little M: Invited Speaker *Functional Genomics in Inherited Kidney Disease*, Renal Genetics Symposium, ANZSN satellite meeting, Canberra, September 2015

Little M: Invited Speaker *Rebuilding the kidney from stem cells*, Ausbiotech, Melbourne, October 2015

Little M: Invited Speaker *Kidney in a dish: the promise of stem cells*, Ausbiotech Public Symposium, Melbourne, October 2015

Little M: Invited Speaker *Self-organising kidneys: how does this happen?*, 112th International Titisee Conference, Organoids: modelling development and disease in 3D culture, Titisee, Germany, October 2015

Little M: Invited Speaker *Modelling Genetic Kidney Disease Using Patient- Derived and CRISPR Cas9-Generated Mutant Pluripotent Stem Cells*, American Society for Nephrology, San Diego, November 2015

Little M: Invited Speaker *The directed differentiation from pluripotent cells to kidney cells*, Joint Congress of the IPITA-IXA-CTS, State-of-the-Art Workshop: Organ Engineering, Melbourne, November 2015

Mason EA: Invited Speaker *Gene expression variability as a unifying element of the pluripotency network and stem cell subpopulations*, Keystone Symposia - Transcriptional and epigenetic influences on stem cell states, Colorado, USA, 2015

Munsie M: Invited Speaker *The big business of selling stem cells*, Brocher Foundation Workshop - Towards consensus on marketing and regulation of emerging and unproven stem cell treatments, Geneva, Switzerland, 2015.

Nielsen LK: Keynote Speaker *Bits, Bugs and Bucks – in silico Biotechnology*, 1st International Conference on Metabolic Sciences (ICMS1), Shanghai, China, 2015.

Nielsen LK: Invited Speaker *Large scale kinetic models*, ME Summit, Beijing, China, 2015.

Nielsen LK: Invited Speaker *Large scale kinetic models*, UQ-TJU SynBio workshop, Tianjin, China, 2015.

Nielsen LK: Invited Speaker *Modelling the Warburg effect in animal cells*, First Meeting of Chilean scientists in Brisbane, Brisbane, Australia, 2015.

Nielsen LK: Invited Speaker *Opening Pandora's box: systems biology for industrial microbes*, ASM 2015, Canberra, Australia.

Nielsen LK: Invited Speaker *Opening Pandora's box: systems biology for industrial microbes*, Novozymes, Denmark, 2015.

Nielsen LK: Invited Speaker *Multi-tissue genome scale modelling: toward understanding plant metabolism at an organismal level*, Bayer, Ghent, Belgium, 2015.

Nielsen LK: Invited Speaker *Opening Pandora's box: systems biology for industrial microbes*, KAIST, Korea, 2015.

Nielsen LK: Invited Speaker *Bits, Bugs and Bucks – in silico Biotechnology*, Tianjin Institute of Industrial Biotechnology CAS, China, 2015.

Nielsen LK: Invited Speaker *Opening Pandora's box: systems biology for industrial microbes*, Tsinghua University, China, 2015.

Nielsen LK: Invited Speaker *Genome scale metabolic and regulatory network modelling in higher eukaryotes*, Peking University, China, 2015.

Ng E: Invited Speaker 44th Annual meeting of the International Society for Experimental Hematology, Kyoto, Japan, 2015.

Ng E: Invited Speaker Stem Cells in the Hunter Valley, ASSCR 2015, NSW, Australia.

Nielsen L: Invited Speaker *Opening Pandora's box: systems biology for industrial microbes*, ASM 2015, Canberra, Australia.

Pébay A: Invited Speaker 24 hour Stem Cell Event, Invitrogen, Online Conference, 2015

Pébay A: Invited Speaker Retina Australia National Congress, Melbourne, Australia, 2015

Pébay A: Plenary Speaker RANZCO meeting, Wellington, New Zealand, 2015

Pera MF: Invited Speaker Young Investigators Symposium, Stem Cell Society of Singapore, Singapore, 2015.

Pera MF: Invited Speaker International Society of Stem Cell Research Stockholm, Sweden, 2015.

Polo J: Invited Speaker American Association for Cancer Research, Philadelphia, USA, 2015.

Polo J: Invited Speaker Stem Cell Symposium, ComBio, Melbourne, Australia, 2015.

Rosenthal N: Plenary Speaker Keystone Symposia Non-Myocyte Cardiac Meeting, Colorado, USA, 2015.

Rosenthal N: Keynote Speaker Child X Conference, Stanford University, USA, 2015.

Rosenthal N: Keynote Speaker Think X Symposium, LKC School of Med, Singapore, 2015.

Rosenthal N: Plenary Speaker Workshop on Cardiomyocyte Biology, Ascona, Switzerland, 2015.

Rosenthal N: Invited Speaker Randall Lecture, Kings College, London, UK, 2015.

Rosenthal N: Plenary Speaker International symposium on stem cell therapy and cardiovascular innovations, Madrid, Spain, 2015.

Rosenthal N: Plenary Speaker British Society for Gene and Cell Therapy Conference, Glasgow, UK, 2015.

Rosenthal N: Keynote Speaker EMBL Australia PhD Course, Perth, Australia, 2015.

Rosenthal N: Plenary Speaker MDIBL Regenerative Biology Symposium, Bar Harbor, USA, 2015.

Rosenthal N: Keynote Speaker AAS Thinktank, Sydney, Australia, 2015.

Rosenthal N: Keynote Speaker BHF Centre of Regenerative Medicine, Annual Oxbridge Meeting, UK, 2015.

Takasato M: Invited Speaker *The directed differentiation from pluripotent cells to kidney cells*, Transplantation Science Symposium, Cutting Edge in Organ Replacement, Lorne, Australia, 2015.

Wells C: Invited Speaker *Integrative vs meta-analysis approaches to biological networks*, Networks in Biological Sciences, Singapore (The Protein Network Workshop), June 2015

Wolvetang E: Invited Speaker *Mission Massimo - Diagnosis to Therapy*, Global Leukodystrophy Initiative consortium meeting, Washington, USA, 10 Jan 2015

Wolvetang E: Keynote Speaker *Functional genomics approaches to human neurological diseases*, Australian Acadamy of Science Australia-China symposium, Melbourne, Australia, 2015.

Wolvetang E: Keynote Speaker *Functional genomics of trisomy 21*, Human Genetics Society of Australia, 12th symposium, Adelaide, Australia, 2015.

Wolvetang E: Invited Speaker *Human functional genomics*, The International Bioeconomy Conference, Ministry of Science and Technology, Tianjin, China, 2015. Oral

Oral Presentations

Asli N. *Cell Cycle and Metabolism at the Heart of Cardiac Regeneration* 23rd ASMR NSW Annual Scientific Meeting Sydney, Australia

Asli N. *PDGF-mediated metabolic drive as a novel cell cycle regulatory pathway in cardiac mesenchymal stem cells* 15th Australian Cell Cycle Meeting Sydney, Australia

Bosman A. *Using Induced Pluripotent Stem Cells (iPSC) to Uncover the Mechanisms Underlying an Enhanced Risk of Atrial Fibrillation* 23rd Annual St Vincent's Campus Research Symposium Sydney, Australia

Bosman A. *Using Induced Pluripotent Stem Cells for Modelling Disease* St Vincent's Centre for Applied Medical Research Seminar Series Sydney, Australia

Cornwell J *Modelling intrinsic and extrinsic control of single cell fates* Stem Cells Australia Cardiac Theme Meeting Sydney, Australia

Dottori M. *Building the nervous system in vitro, from floor plate to roof plate with human pluripotent stem cells* Cell Reprogramming Australia Annual Meeting Brisbane, Australia

Elliott D. *Pluripotent stem cell models of human heart development and disease* Australian Network of Cardiac and Vascular Developmental Biologists Adelaide SA, Australia

Elliott D. High Blood Pressure Research Council Australia Melbourne, Australia

Fonoudi H. *Investigating Early Cardiac Development in Patients with Hypoplastic Left Heart using Induced Pluripotent Stem Cells* St. Vincent's Symposium Sydney, Australia

Fonoudi H. *Investigating Cardiac Development and Function in iPS cells generated from patients with hypoplastic left heart* Stem Cell Australia Cardiac Theme Meeting Sydney, Australia

Fonoudi H. *Investigating genetic causation of Hypoplastic Left Heart syndrome using induced pluripotent stem cells* NSW and ACT Cell & Developmental Biology Meeting Sydney, Australia

Forte E. *Chase and trace cardiac MSCs function* Stem Cells Australia Cardiac Theme Meeting Sydney, Australia

Hudson J. Australian Network of Cardiac and Vascular Developmental Biologists Adelaide SA, Australia

Hudson J. 2015 Combined SCA ASSCR Meeting Hunter Valley NSW, Australia

Janbandhu V. *Physiological functions of adult cardiac-resident colony-forming units – fibroblast (cCFU-Fs)* Fondation Leducq Annual Meeting Paris, France Young Scientist Presentation

Janbandhu V. Stem Cell Australia Cardiac Theme Meeting Sydney, Australia

Janbandhu V. *Metabolic regulation of cCFU-F (cardiac-resident colony-forming units – fibroblast) functions* 23rd Annual St Vincent's Campus Research Symposium Sydney, Australia Winner of the best Fast Forward Presentation

Kikuchi K. *A subpopulation of T cells promoting tissue regeneration in zebrafish* Australian Network of Cardiac and Vascular Developmental Biologists Adelaide SA, Australia

Korn O. *Stemformatics and RNASeq AGRF Next Gen SIG* Queensland, Australia

Mason EA. *Gene expression variability as a unifying element of the pluripotency network and stem cell sub-populations* Monthly Seminar Series: Ichan School of Medicine Mt Sainai Hospital New York, USA

Palpant N. *Human Cardiac, Endothelial and Blood Lineages are Controlled by Gradients of Activin A, BMP4, and Wnt/Đ-Catenin Signaling* International Society for Heart Research Seattle, USA

Palpant N. *Mechanisms of mesoderm cell fate determination and inter-conversion using human pluripotent stem cells* Ascona International Workshop on Cardiomyocyte Biology Monte Verità, Ascona, Switzerland

Palpant N. *The epigenetic and transcriptional landscape of mesoderm progenitor cells identifies HOPX as a novel regulator of hemogenic endothelium* Institute for Stem Cell and Regenerative Medicine Symposium Washington, USA

Palpant N. *Mechanisms of Cell Fate Determination in Mesoderm Development* Australian Network of Cardiac and Vascular Developmental Biologists Adelaide SA, Australia

Palpant N. *Mechanisms of Cell Fate Determination in Mesoderm Development* 2015 Combined SCA ASSCR Meeting Hunter Valley NSW, Australia

Porrello E. *Multicellular transcriptome analysis of neonatal heart development and regeneration* Australian Network of Cardiac and Vascular Developmental Biologists Adelaide SA, Australia

Porrello E. Cardiac Society of Australia and New Zealand and International Society for Heart Research Melbourne, Australia

Porrello E. Australian Physiological Society Hobart, Australia

Porrello E. COMBIO Melbourne, Australia

Taoudi S. *Hematopoiesis during embryonic development* Australian Network of Cardiac and Vascular Developmental Biologists Adelaide SA, Australia

Wells C. *MSC Signature* ISSCR annual meeting Stockholm, Sweden Poster & Oral

WystubLis K. *microRNAs determining cardiomyocyte identity* Stem Cell Australia Cardiac Theme Meeting Sydney, Australia Oral

Poster Presentations

Abeygunawardena D. *Lineage commitment of cardiac MSCs* Stem cells Australia Cardiac Theme Meeting Sydney, Australia

Alshawaf A, Chana G, **D'abaco G,** Boyd C, Everall I, Skafidas S, & **Dottori M.** *Human pluripotent stem cells based modelling of brain development, function and associated disorders* the 1st Saudi Students Conference in Australia Melbourne, Australia

Alshawaf A, Qiu W, Petrou S, **Viventi S, D'abaco G,** Chana G, Everall I, Skafidas S, & **Dottori M.** *Functional characterization of cultured neurons derived from human pluripotent stem cells* ASSCR Annual Meeting Hunter Valley, NSW, Australia

Alshawaf A, Qiu W, **D'abaco G,** Chana G, Everall I, Skafidas S, & **Dottori M.** *Functional In Vitro Modelling Of The Brain Using Human Pluripotent Stem Cells; A Platform to Study Autism and Schizorphenia* the Students of Brain Research (SOBR) Sympsium Melbourne, Australia

Crombie DE, Kulkarni T, Sivakumaran P, Van Bergen N, **Wong RCB,** Evans-Galea M, Bird MJ, Minami I, Bullough D, Ozsolak F, Lim S, Corben LA, **Dottori M,** Hewitt AW, Trounce IA, Nakatsuji N, Delatycki MB, **Pera MF,** & **Pébay A.** *Screening of phenotypic abnormalities in Friedreich's ataxia-induced pluripotent stem cell-derived cardiomyocytes* International Ataxia Research Conference Windsor, UK

Davidson KC, Conquest A, Liang H, Kashyap A, Hewitt AW, Mountford S, Cain M, **Wong RCB,** Thompson P, **Pera**

MF, Guymer RH, & **Pébay A.** *Developing induced pluripotent stem cell-based models of retinal degenerative diseases that combine genetics, ageing, and environmental risk factors in a dish.* Cell Reprogramming Australia Annual Conference Brisbane, Australia

Fonoudi H, Bosman A, Blue G, Winlaw D**, Harvey RP.** *Investigating Early Cardiac Development in Patients with Hypoplastic Left Heart using Induced Pluripotent Stem Cells* St Vincent's Campus Research Symposium Sydney, Australia

Fonoudi H, Bosman A, Blue G, Winlaw D, **Harvey RP.** *Investigating early cardiac development in patients with Hypoplastic Left Heart using induced pluripotent stem cells* Basic Cardiovascular Science 2015 New Orleans, USA

Fonoudi H, Bosman A, Blue G, Winlaw D, Harvey RP. *Investigating early cardiac development in patients with Hypoplastic Left Heart using induced pluripotent stem cells* ASSCR Annual Meeting Hunter Valley, NSW, Australia

Forte E. *Physiological Role of Adult Cardiac Colony-forming Unit Fibroblasts* Basic Cardiovascular Science 2015 New Orleans, USA

Forte E. *Physiological role of endogenous adult cardiac colony-forming unit fibroblasts* St Vincent's Campus Research Symposium Sydney, Australia

Gill KP, Waugh HS, Lidgerwood GE, **Wong RCB,** Hewitt AW, & **Pébay A.** *Primary retinal ganglion and pigment epithelium cell isolates from human donor tissue allow for gene expression comparison between native and pluripotent stem cell derivatives* ASSCR Annual Meeting Hunter Valley, NSW, Australia

Gill KP, Hung SCS, Needham K, Hewitt AW, **Pébay A,** & **Wong RCB.** *Differentiation and enrichment of retinal ganglion cells from human embryonic stem cells* 13th ISSCR Annual Meeting Stockholm, Sweden

Hernández D, Sivakumaran P, Millard R, **Wong RCB,** Hewitt AW, Liang H, **Hung SSC, Pébay A,** Shepherd RK, Lim SY, & Dusting GJ. *Cardiac regeneration by differentiation and maturation of cardiomyocytes from human induced pluripotent stem cells: the benefits of short- and long-term electrical stimulation* High Blood Pressure Research Council of Australia Annual Scientific Meeting Melbourne, Australia

Hernandez D, Sivakumaran P, Millard R, **Wong RCB,** Hewitt AW, Liang H, **Hung SSC, Pébay A,** Shepherd RK, Lim SY, & Dusting GJ. *Cardiac differentiation and maturation of cardiomyocytes from human induced pluripotent stem cells: the benefits of short- and long-term electrical stimulation* ASSCR Annual Meeting Hunter Valley, NSW, Australia

Hobbs R. *Characterization of molecular mechanisms defining germline progenitor cell heterogeneity and fate* ISSCR Annual conference Stockholm, Sweden

Hung SCS, Wong RCB, Sanij E, Jackson S, Pearson RB, Hannan RD, Hewitt AW, Nayagam B, & **Pébay A.** *Generation of an immortalised human retinal ganglion cell (RGC) line from pluripotent stem cells* 13th ISSCR Annual Meeting Stockholm, Sweden

Hung SCS, Liu RGS, **Wong RCB, Pébay A,** & Hewitt AW. *Transgenic tools for optimizing in vivo CRISPR/Cas delivery and mutation correction* Genome Engineering Workshop 3.0 Boston, USA

Janbandhu V. *Metabolic regulation of cCFU-F (cardiac-resident colony-forming units – fibroblast) functions* 23rd Annual St Vincent's Campus Research Symposium Sydney, Australia

Lidgerwood GE, Lim SY, **Crombie DE,** Ali R, **Gill KP,** Hernández D, Kie J, Conquest A, Waugh HS, **Wong RCB,** Liang H, Hewitt AW, **Davidson KC,** & **Pébay A.** *Defined conditions for the induction and expansion of human pluripotent stem cell-derived retinal pigmented epithelium* ASSCR Annual Meeting Hunter Valley, NSW, Australia

Mason EA, Hough SR, Mar J, **Laslett A,** Quackenbush J, **Wolvetang E, Wells C,** & **Pera, MF.** *Gene expression variability as a unifying element of the pluripotency network and stem cell subpopulations* Keystone Symposia - Transcriptional and epigenetic influences on stem cell states Colorado, USA

Mason EA, Hough SR, Mar J, **Laslett A,** Quackenbush J, **Wolvetang E, Pera MF,** & **Wells C.** *A minimal co-expression network describes transition through the stem cell hierarchy* ISSCR Annual meeting Stockholm, Sweden

Mason EA, Hough SR, Mar J, **Laslett A,** Quackenbush J, **Wolvetang E, Pera MF,** & **Wells C.** *A minimal co-expression network describes transition through the stem cell hierarchy* ASSCR Annual Meeting Hunter Valley, NSW, Australia

McCaughey T, Ali R, Chen C, Fenwick E, Rees G, Vickers JC, Mathew Summers M, Kearns LS, MacGregor C, **Munsie M, Pébay A,** & Hewitt AW. *Avoiding HeLa: Incorporating a video animation to improve Informed Consent for Induced Pluripotent Stem Cell Research* HeLEX, Oxford, UK Oxford, UK

McDonald NLV, Briggs EA, **Wong RCB, Pébay A,** Hewitt AW, & Cook AL. *Defining conditions for a human stem cell model of the glaucoma-affected trabecular meshwork cell lineage* ASSCR Annual Meeting Hunter Valley, NSW, Australia

Mattei C, D'Abaco G, Nasr B, **Dottori M, Alshawaf A,** Chana G, Everall I, & Skafidas S. *Graphene foam is a valid 3D scaffold for culturing hESC-derived cortical progenitor cells* ASSCR Annual Meeting Hunter Valley, NSW, Australia

Pébay A, Wong RCB, Kulkarni T, Sivakumaran P, Van Bergen N, Evans-Galea M, Bird MJ, Minami I, Bullough D, Ozsolak F, Lim S, Corben LA, **Dottori M,** Hewitt AW, Trounce IA, Nakatsuji N, Delatycki MB, **Pera MF,** & **Crombie DE.** *Phenotypic abnormalities in Friedreich's ataxia-induced pluripotent stem cell-derived cardiomyocytes* 13th ISSCR Annual Meeting Stockholm, Sweden

Sivakumaran P, Hernández D, **Wong RCB,** Taylor C, Hewitt AW, Liang H, **Hung SSC,** Dusting GJ, **Pébay A, & Lim SY.** *Mdivi-1, a mitochondrial fission protein DRP1 inhibitor, directs cardiac differentiation of human induced pluripotent stem cells* ASSCR Annual Meeting Hunter Valley, NSW, Australia

Viventi S, Alshawaf A, Frausin S, Qiu W, **D'Abaco G, B**ird M, Frazier A, **Crombie D,** Thorburn D, **Pebay A,** Thompson L, Skafidas S, & **Dottori M.** *In vitro and in vivo characterization of sensory neurons derived from Friedreich Ataxia induced pluripotent stem cells* ASSCR Annual Meeting Hunter Valley, NSW, Australia

Viventi S, Alshawaf A, Frausin S, Qiu W, **D'Abaco G,** Bird M, Frazier A, **Crombie D,** Thorburn D, **Pebay A,** Thompson L, Skafidas S, & **Dottori M.** *In vitro and in vivo characterization of sensory neurons derived from Friedreich Ataxia induced pluripotent stem cells.* FARA Friedreich Ataxia National Research Review Meeting Melbourne, Australia

Wells C. *MSC Signature* ISSCR Annual meeting Stockholm, Sweden

Wong RCB, Hung SSC, Van Bergen N, Jackson S, Lim SY, Hernandez D, **Gill KP, Lidgerwood GE, Mackey D,** Liang H, Kearns L, Hewitt A, Trounce I, & **Pébay A.** *Using pluripotent stem cells to model optic neuropathies* 13th ISSCR Annual Meeting Stockholm, Sweden

Advisory boards, panelists, invites

Bartlett P: Invited Facilitator, China - Australia Collaboration *Where to from here?*, Australia - China Symposium on Neuroscience, Werribee, Australia, 2015.

Graham RM: Member, Board of Scientific Governors MacTel Study, Lowy Medical Research Institute, San Diego. USA.

Graham RM: Member, Board of Scientific Governors MacTel Study, Lowy Medical Research Institute, New York, USA.

Munsie M: Chair ISSCR Closer Look at Stem Cells taskforce

Munsie M: Member ISSCR Guidelines on Clinical Translation taskforce and ethics committee

Munsie M: Chair ASSCR Policy, Ethics and Translation Sub-Committee

Munsie M: Chair Centre for Eye Research Australia Vision Restoration Program

Munsie M: Member Praxis Australia Research Training Advisory Committee

Nielsen L: Scientific Advisory Board Biochemical and Molecular Engineering XIX Conference, July 12-16, 2015, Los Cabos, Mexico

Pera MF: Human Science Frontiers Program Grantees Meeting, La Jolla CA, USA.

Pera MF: Clerk and Member, Board of Directors, ISSCR

Meetings organised and/or chaired

Chong J: Member of organising committee and session chair *Theo Murphy Think Tank The Stem Cell Revolution: Lessons for Australia*, Australian Academy of Sciences, Sydney, Australia.

Dottori M: Organiser Neuroscience Stream at COMBIO Meeitng

Elefanty A: Conference Organizer, session chair, panel moderator *Stem Cells: Basic Biology to Disease Therapy,* Cold Spring Harbor-Asia and ISSCR International Symposium, Suzhou, China.

Harvey R: Co-convenor *Theo Murphy Think Tank The Stem Cell Revolution: Lessons for Australia*, Australian Academy of Sciences, Sydney, Australia.

Laslett A: Co-Organizer Annual meeting of Cell Reprogramming Australia, Brisbane, Australia.

Little M: Member of organizing committee International Workshop for Developmental Nephrology, Snowbird, Utah, USA.

Little M: Chair Joint Congress of the IPITA-IXA-CTS, State-of-the-Art Workshop: Organ Engineering, Melbourne

Munsie M: Member of organizing committee and session chair *Theo Murphy Think Tank The Stem Cell Revolution: Lessons for Australia*, Australian Academy of Sciences, Sydney, Australia.

Munsie M: Co-Organizer Annual meeting of Cell Reprogramming Australia, Brisbane, Australia.

Munsie M: Member, Co-convenor and session chair AusBiotech Regenerative Medicine Symposium

Munsie M: Session Chair Ethics and Legal session at Australasian Society for Stem Cell Research Annual Conference

Munsie M: Session Chair High hopes, high risks? Community expectations in setm cell treatments in borderless healthcare

Nielsen L: Steering Committee memberECI Cell Therapy Conference

Carmel O'Brien: Co-Organizer Annual meeting of Cell Reprogramming Australia, Brisbane, Australia.

Pera MF: Member of organising committee and session chair *Theo Murphy Think Tank The Stem Cell Revolution: Lessons for Australia*, Australian Academy of Sciences, Sydney, Australia.

Pera MF: Chair ISSCR Membership Committee

Polo J: Panel Chair*Epigenetics 2015*, Hobart, Australia.

Polo J: Co-Organizer Annual meeting of Cell Reprogramming Australia, Brisbane, Australia.

Wolvetang E: Co-Organizer Annual meeting of Cell Reprogramming Australia, Brisbane, Australia.

Community Activities

We would like to acknowledge and thank our partners and participants for their support of our 2015 community outreach activities.

StemCells@UQ, Brisbane, Queensland - **Andrew Laslett, Christine Wells, Ernst Wolvetang, Melissa Little** and **Mirella Dottori**

Translating Stem Cell Research Into Real Health and Economic Benefits, Brisbane, Queensland - **Christine Wells, Ernst Wolvetang, Martine Pera** and **Peter Gray**

ISSCR *'Stem cell research: promise, progress and hype'* media briefing session, Stockholm, Sweden - **Megan Munsie**

Bodies, borders and biologicals: ethical considerations of medical tourism - Melbourne, Victoria - **Megan Munsie**

Science in cinema, Blade Runner, Melbourne, Victoria - **Megan Munsie**

Selling stem cells: the need to reconcile hype, hope & evidence, Melbourne, Victoria - **Megan Munsie**

Brainoids: Growing a brain in a dish, Melbourne, Victoria - **Martin Pera, Mirella Dottori** and **Trevor Kilpatrick**

Retina Australia, Melbourne Victoria, - **Alice Pebay** and **Megan Munsie**

Made to Order: can science regenerate body parts?, Melboure, Victoria - **David Haylock, James Godwin, Megan Munsie** and **Melissa Little**

Hype, hope or reality - can we make eggs or sperm from stem cells?, Melbourne, Victoria - **Megan Munsie** and **Robin Hobbs**

Lost in evolution: How research is unlocking the mystery of regenerative medicine, Melbourne, Victoria - **Peter Currie, Mirana Ramialison, James Godwin** and **Megan Munsie** - with Convergence Science Network

Graeme Clarke Oration Schools Program with Paul Nurse, Melbourne, Victoria - **Megan Munsie**

MND NSW Ask the Experts Forum, Sydney, NSW - **Megan Munsie**

The Stem Cell Podcast - Ep 43: "A Closer Look at Stem Cells" - **Megan Munsie** and Mario D'Cruz

Media Coverage

Press Releases

Includes those related to Stem Cells Australia research released by our partners.

Unraveling the biological mystery of how cells regulate their fate 13 February 2015.

Call for urgent action to curb sale of unproven stem cell treatments in Australia 03 March 2015.

Selling stem cells: a need to reconcile hype, hope and evidence 09 September 2015.

Realising high hopes for the new wave of medicine in conjunction with AusBiotech 16 September 2015.

Melbourne to host major international stem cell meeting 12 November 2015.

Experts warn more testing needed before stem cells can be used to make sperm and eggs 19 November 2015.

Media Releases

Stories related to Stem Cells Australia's research and other activities.

One step back, 10 steps forward for stem cells The Weekend Australian, 3-4 January 2015

Stem cell therapy regulation raises questions for patients and practitioners ABC The World Today, 9 January 2015

Stem cell tourism in Australia 2SER Radio, 27 January 2015

Stem cell clinics on trial Austrlian Doctor, 2 February 2015

New stroke treatment gives brain repair hope Herald Sun, 3 February 2015

Timely stroke treatment Townsville Bulletin, 4 February 2015

Brain repair hope Herald Sun, 4 February 2015

Future cancer treatments may target DNA 'organisers' The Australian, 13 February 2015

FANTOM5 project discovers general rules governing how cells change Phys.org, 13 February 2015

Discovery of brain pathway could lead to ways to prevent blindness Sydney Morning Herald/ The Age, 17 February 2015

Is a loophole in stem cell law helping new therapy to thrive, or allowing dubious science? Sydney Morning Herald/ The Age, 5 April 2015

Australian researchers help find new way to regrow heart muscle Sydney Morning Herald, 6 April 2015

Heart muscle cells regrow in medical research breakthrough The Guardian, 7 April 2015

Helping the heart repair itself after a cardiac arrest: researchers say they've worked out how ABC Radio AM, 7 April 2015

Scientific breakthrough could give new hope to heart-attack patients SBS, 7 April 2015

Stem-cell therapy needs tighter regulation, say experts Australian Financial Review, 8 April 2015

We have expertise to make human-animal embryos, but should we? The Australian, 7 May 2015

Stem cell tourism continues to gain popularity despite the risks Courier Mail, 30 May 2015

Stem cell loophole must be closed Australasian Science, 1 June 2015

Stem cell therapy dilemma ABC Lateline, 8 June 2015

Scientists warn stem cell miracle cure Ny Teknik Sweden, online news 26 June 2015

Legal loophole exploited for private stem cell trials The Australian, 11 August 2015

Travellling overseas for medical treatment ABC Radio QLD, 13 August 2015

Stem cells from human skin turned to kidney tissue The Guardian, 7 October 2015

Researchers Grow Kidney-Like Organs in Laboratory Wall Street Journal, 7 October 2015

Kidneys in a dish: Melbourne researchers use stem cells to grow tiny organs ABC The World Today, 8 October 2015

Lab-grown human kidney a breakthrough for medical researchers Sydney Morning Herald/ The Age, 8 October 2015

Stem cell kidneys offer new hope for organ replacement The Australian, 8 October 2015

Human kidneys grown in a dish could change the way drugs are tested News.com.au, 8 October 2015

How the brain responds to damage ABC Radio National Science Show, 12 October 2015

Stem cell experts urge ethical debate over embryo creation ABC News, 13 October 2015

Australia's First Single-Cell Genomics Centre of Excellence Opens at Monash Health Translation Precinct Business Wire, 3 November 2015

Professor Perry Bartlett honoured with top biomedical prize ABC PM, 11 November 2015

CSL Florey medal recipient Professor Perry Bartlett ABC Online, 11 November 2015

Brisbane brain scientist wins award for contribution to 'last frontier' Brisbane Times, 11 November 2015

How regenerative medicine and the use of stem cells is becoming big business Business Insider, 3 December 2015

Notes

Financial Statement

Financial Statement For Calendar Year January To December 2015

STEM CELLS AUSTRALIA	2015	Project to date
ARC Funds	3,446,192.00	14,782,493.92
Other Contributions	2,043,865.72	8,379,218.92
Total Income	5,490,057.72	23,161,712.84
Salaries and oncosts	3,262,264.81	12,951,100.66
Consumables and other costs	2,564,355.62	8,473,058.61
Total Expenses	5,826,620.43	21,424,159.27
Net Activity for the year	(336,562.71)	1,737,553.57
Carry over balance	2,074,116.28	
Balance at Dec 2015	1,737,553.57	1,737,553.57

I certify that:
a) The figures reported above are true and correct in every particular to the best of my knowledge and having made all due enquiries.

Mike Pham
Research Accountant
Research Accounting Services

12/04/16
Date

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