Media Release

Stem Cell Scientists First to be Awarded Victoria-California Collaborative Grants to Drive Stem Cell Research Forwards

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Victorian stem cell scientists from the Australian Stem Cell Centre, Monash University and the Florey Neuroscience Institutes are the first recipients of collaborative grants under the Victoria-California Stem Cell Alliance.

Four collaborative stem cell projects involving researchers from the Australian Stem Cell Centre, Monash University and the Florey Neuroscience Institutes are the first to be funded under the International Alliance between the State of Victoria and the California Institute of Regenerative Medicine (CIRM).

The projects were announced late last night (AEST) by Victoria's Minister for Innovation, Gavin Jennings at BIO2009 in Atlanta, Georgia.

The Victorian collaborators represent some of the best stem cell scientists in the world. Their research focus varies from studying the immune system in conjunction with stem cells to prevent rejection, to harnessing the abilities of human embryonic stem cells and induced pluripotent stem cells (iPS cells) and their potential for growth and differentiation into all the cells of the body.

The successful projects were submitted to the CIRM Early Translational Research Awards that are designed to move promising basic research in stem cell science toward the clinic for eventual patient benefit. A total of 72 applications were received at CIRM, with a total of 15 selected to receive funding at this time, of which four are Victorian collaborative projects.

The four successful projects are:

Methods for detection and elimination of residual human embryonic stem cells in a differentiated cell product.
Andrew Elefanty and Ed Stanley, Monash University and Novocell Inc.

Neural Stem Cells as a Developmental Candidate to Treat Alzheimer's Disease.
Richard Boyd, Australian Stem Cell Centre and Monash University and University of California, Irvine

Ensuring the safety of cell therapy: a quality control pipeline for cell purification and validation.
Andrew Laslett, Australian Stem Cell Centre and Scripps Research Institute

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Developmental Candidates" for Cell-Based Therapies for Parkinson's Disease.

Clare Parish and Colin Pouton, Florey Neuroscience Institutes and Monash University and Burnham Institute for Medical Research

QUOTES:

“Considered in combination, these projects will accelerate the application of stem cell science to treating illnesses in patients, by addressing current safety issues and other hurdles to giving stem cells or their products to sick people and understanding the immune response to stem cell treatments.” said Professor Graham Macdonald, Chair of the Australian Stem Cell Centre. He added “in addition, the projects stand to add to our understanding of the body’s mechanisms for directing tissue development from stem cells and how stem cells themselves control the activities of surrounding cells. This will in turn lead us to new mechanisms for understanding and treating disease.”

"Monash is an internationally focused university that supports intricate research programs on the most pressing biomedical issues. These exciting joint projects with California are a triumph of collaboration between the brightest experts in the world that will help to tackle a range of debilitating illnesses," said the University’s Deputy Vice Chancellor (Research), Professor Edwina Cornish.

“These projects bring together scientists who each have a special expertise within stem cell research and focuses on collaboration where each step undertaken fits together like a jigsaw puzzle. It will bring basic science much closer to treatments for debilitating neurological disorders,” said Professor Malcolm Horne, Deputy Director, Florey Neuroscience Institutes.

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The Minister for Innovation’s press release can be found here.

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About the Projects

Neural Stem Cells as a Developmental Candidate to Treat Alzheimer Disease
Australian Stem Cell Centre, Melbourne and the University of California, Irvine
Professor Richard Boyd (Australia) and Frank LaFerla (California) will lead the project.

This project aims to determine whether neural stem cells can be translated from the bench to the clinic as a therapy for Alzheimer 's disease. Alzheimer's is a progressive neurodegenerative disorder that affects over 4.5 million Americans and over 500,000 Australians. There are currently no effective therapies for the treatment of Alzheimer's. Existing treatments provide minor symptomatic relief but these are often associated with severe side effects. Multiple strategies are likely to be needed to prevent or treat Alzheimer's disease, including the utilisation of cell-based approaches. This project builds on extensive preliminary data that support the feasibility of neural stem cell-based therapies for the treatment of Alzheimer's disease.

The project leaders will use human embryonic stem cells that are transformed into human neural stem cells, and then will test their ability to improve cognitive recognition function in an animal model of Alzheimer's disease in the laboratory. However, a challenge in successfully treating patients with these therapeutic cells is the very high likelihood that they will be rejected by the patient's immune system.

The multi-disciplinary team of scientific leaders from the fields of stem cell biology, animal modelling, neurodegeneration, immunology, genomics and Alzheimer's disease clinical trials will collaborate on this early translational study, aiming to develop a novel treatment for Alzheimer's disease. The Victorian component, led by Professor Richard Boyd, will apply their technologies for re-educating the immune system to develop novel strategies for ensuring long-term acceptance of the stem cells transplants, without the need for prolonged usage of immunosuppressive drugs.

About Professor Richard Boyd

Professor Richard Boyd is the Director of Monash Immunology and Stem Cell Laboratories, where he leads a laboratory of approximately 25 people. He is also Chief Scientific Officer of UK AIM-listed biotechnology company Norwood Immunology, who have supported the translation of his research to clinical trials in Australia and the US.

Professor Boyd's research has focused on the formation and growth of the immune system and his group were the first to grow an organ from stem cells. They identified epithelial stem cells in the embryonic mouse, which could form a thymus after transplantation. His laboratory established an internationally recognised leadership position in understanding the nature and function of the thymic microenvironment. Recently they have developed technologies for reversing the age-associated degeneration of the immune system, particularly the thymus, including making haematopoietic stem cell transplantation more efficient.

Professor Boyd has published over 200 papers and for over 25 years has had a major role in educating undergraduate and postgraduate students (he has supervised over 65 Bachelor of Science Honours candidates and 32 Doctor of Philosophy candidates) at Monash University. He has also given many public lectures on immunology and stem cells, and over 500 scientific presentations at national and international conferences, and at research institutes. In 2004, the collaboration between Professor Boyd's laboratory and Norwood Immunology was awarded an Australian Government Business / Higher Education Round Table award for outstanding achievement in research and development, and education and training. Professor Boyd's research is partially supported by the Australian Stem Cell Centre.

Ensuring the safety of cell therapy: a quality control pipeline for cell purification and validation

Australian Stem Cell Centre, Melbourne and the Scripps Research Institute, La Jolla

The project will be led by Dr Andrew Laslett (Australia) and Dr Jeanne Loring (California).

Human embryonic stem cells have great potential in cell-based therapies due to their pluripotent nature (they can become any desired human cell type). To achieve this potential, more precise information regarding their safety, stability and controlled differentiation (changing into various cell types) is required. Demonstration of safety in a cell-based therapy Phase I clinical trial is a critical stage in advancing new effective treatments for patients.

The project focuses on the design of a quality control pipeline that will vastly improve the ability of scientists and clinicians to identify and remove cells that are likely to become toxic or cancerous prior to transplantation. This collaboration will develop products, tools and quality control measures, including methods to identify and remove unwanted cells from mixed cell populations.

This project addresses safety concerns about pluripotent stem cell therapies expressed by the US regulator (the Food and Drug Administration), specifically the possibility that contamination of cell populations by rare undifferentiated human embryonic stem cells may lead to unregulated growth and to tumour formation. Through the development of robust protocols for the detection and removal of unwanted cells, this major barrier to the utilisation of human embryonic stem cell in the clinic can be overcome. The head of the Victorian component, Dr Andrew Laslett, will lead the cell purification studies and work closely with the Monash Antibody Technologies Facility, which will produce the bulk of the antibodies for the project.

About Dr Andrew Laslett

In August 2006 Dr Andrew Laslett was appointed as a Senior Scientist and Group Leader of the Human Embryonic Stem Cell Technology Laboratory at the ASCC. Dr Laslett is also an Honorary Senior Lecturer in the Department of Anatomy and Developmental Biology at Monash University. Prior to joining the ASCC, he was a Senior Research Fellow in the Laboratory of Embryonic Stem Cell Biology, Centre for Reproduction and Development, Monash Institute of Medical Research, Monash University.

Dr Laslett obtained his BSc (Hons) and PhD from Monash University prior to undertaking postdoctoral positions in Hong Kong and Philadelphia, USA.

His previous research focused on cellular interactions in the male reproductive system and gave him excellent grounding in cell culture and molecular biological techniques. Since 2001, Dr Laslett has focused on elucidating the complex biology of human embryonic stem cells, and has examined methods for the differentiation of human embryonic stem cells to renal progenitor cells. More recently he has compared human embryonic stem cells to human induced pluripotent stem cells. Dr Laslett's research has been both nationally and internationally recognised for increasing the basic understanding of human embryonic stem cells. He leads an independent program as well as having significant national and international collaborations. In September 2007, Dr Laslett was elected as a Board Member and Director of the Australian Society of Medical Research (ASMR). Dr Laslett's research is supported by the Australian Stem Cell Centre, the NHMRC and the NSW / Victorian Government Stem Cell Research Grant Program.

Methods for detection and elimination of residual human embryonic stem cells in a differentiated cell product

Monash University and Novocell Inc., San Diego

The project will be led by Professor Ed Stanley and Professor Andrew Elefanty (Australia) and Dr Justine Cunningham (California).

This project, to be led in Victoria by Professors Ed Stanley and Andrew Elefanty, takes an alternative scientific approach to focus on the question of safety of potential human embryonic stem cells based products. The aim of the project is to establish standardised tests to ensure the safety of human embryonic stem cell based products, with the ultimate goal of contributing to the development of human embryonic stem cell based therapies for insulin dependent diabetes. The project will develop accurate tests to measure teratoma (tumour) formation potential that will contribute to the essential safety data required for the transition of stem cell-based treatments for diabetes into the clinic. The Californian collaborators are led by Novocell Inc., a private company whose focus on stem cell engineering aims to develop insulin producing cells from human embryonic stem cells to treat insulin dependent diabetics.

Contamination of a human embryonic stem cell-derived therapeutic cell product with undifferentiated cells and the ensuing risk of tumor formation is a major safety concern that has significant impact on the development and application of stem cell therapies. This project will develop a sensitive standardised teratoma test as well as a test for biomarkers that will allow for the earlier detection of teratomas. The project also aims to identify compounds that are toxic for human embryonic stem cells, but which spare more differentiated cells to eliminate residual human embryonic stem cells from the resulting cell products. Finally, these methods will come together in the production and testing of a human embryonic stem cell-based treatment for diabetes intended for clinical development.

About Professor Ed Stanley

Professor Ed Stanley is (with Professor Elefanty) Joint Laboratory Head of the Embryonic Stem Cell Differentiation Laboratory of the Monash Immunology and Stem Cell Laboratories. Professor Stanley completed his PhD at the Ludwig Institute for Cancer Research in Melbourne before traveling to the National Institute for Medical Research, Mill Hill, London to undertake post-doctoral studies. During this time he was awarded a Human Frontiers in Science Organization Fellowship and a CJ. Martin Fellowship. Following his stay in London, Professor Stanley returned to the Walter and Eliza Hall Institute (WEHI) of Medical Research in Melbourne to continue his postdoctoral training in developmental biology. In 2000 he was appointed head of the gene targeting facility at WEHI.

About Professor Andrew Elefanty

Professor Andrew Elefanty is the Joint Laboratory Head of the Embryonic Stem Cell Differentiation Laboratory of the Monash Immunology and Stem Cell Laboratories. Professor Elefanty trained as a physician in medical oncology and completed a PhD in leukaemogenesis under the supervision of Professor Suzanne Cory at the Walter and Eliza Hall Institute of Medical Research in 1992. He was awarded an NHMRC Neil Hamilton Fairley travelling fellowship and a Roche travelling fellowship from the Royal Australasian College of Physicians in 1993, taking up a position at the National Institute for Medical Research in London in the laboratory of Professor Frank Grosveld, studying aspects of blood cell development. He returned to the WEHI in 1995 as a Special Fellow, Head of the Developmental Haematopoiesis laboratory.

The Embryonic Stem Cell differentiation Laboratory, headed jointly by Professor Andrew Elefanty and Professor Ed Stanley, moved to Monash University in 2002. The laboratory has focused on human embryonic stem cell differentiation along mesodermal (blood, endothelium and heart) and endodermal (pancreas) lineages. The group has made significant contributions to the field in the culture of human embryonic stem cells, and they have developed a robust system for the efficient differentiation of human embryonic stem cells, complemented by the development of a safe animal product free medium in which human embryonic stem cell differentiation can be reproducibly directed to different lineages by the inclusion of specific growth factors. The group has generated genetically modified human embryonic stem cells lines in which fluorescent reporters have been introduced into key gene loci that allow objective monitoring of in vitro differentiation of embryonic stem cells in a logical, step-wise fashion.

A major goal of their work is to regulate human embryonic stem cell differentiation in order to understand human development, to generate tools for drug discovery, and eventually to provide a source of cells for therapy.

"Developmental Candidates" for Cell-Based Therapies for Parkinson's Disease (PD)

Florey Neuroscience Institutes, Monash University, Melbourne and Burnham Institute of Medical Research, La Jolla:

The project will be led by Dr Clare Parish, Professor Colin Pouton (Australia) and Professor Evan Snyder (California).

This project aims to identify the best candidates for cell-based therapies for Parkinson's Disease using animal models. Parkinson's Disease severely debilitates about two per cent of the US population and approximately 80,000 Australians. The disease results from the progressive loss of a population of cells in the brain (dopamine cells) that release the chemical dopamine that is important for regulating movement. There are currently no effective therapies for the treatment of Parkinson's.

The Californian collaborators have discovered that human neural stem cells (hNSCs) may exert a significant beneficial impact in predictive animal models of actual human Parkinson's disease. While some of the hNSCs differentiate into replacement dopaminergic neurons, much of the therapeutic benefit derived from a stem cell action is due to what is called the 'Chaperone Effect' – whereby hNSC-derived cells that do not become dopamine neurons contribute to the reversal of severe Parkinsonian symptoms. While the ultimate goal may someday be to replace dead dopamine neurons, the Chaperone Effect represents a more tractable near-term method of using cells to address this serious condition. However, many questions remain in the process of developing these cellular therapeutic candidates.

This project aims to answer some of those questions by studying different methods and cells from which to create hNSCs, as well as what other supporting factors such as molecules contribute to this effect. The Victorian team has developed genetic methods for identifying specific precursors of dopaminergic neurons which allows these precursors to be purified for use in implantation experiments. Implantation of committed precursors may represent an improved or complementary approach to hNSC cell therapy of Parkinson's disease. The Victorian team will examine whether early or late dopaminergic progenitors represent the best approach for cell therapy and will provide suitable cells to the Californian team for inclusion in cell therapy studies.

About Dr Clare Parish

Dr Clare Parish is a senior research officer at the Florey Neurosciences Institutes. Dr Parish received her Bachelor of Biomedical Science (Honours), and subsequently her PhD from Monash University. In 2003 she embarked on a 4-year postdoctoral research program at the Karolinska Institute in Stockholm, Sweden – renowned for its annual awarding of the Nobel Prize in physiology or medicine. At Karolinska, Dr Parish studied stem cells biology and their application in Parkinson's disease models.

In 2007 she returned to the Florey Neurosciences Institutes to establish an independent research group. Dr Parish has been awarded a number of national and international awards and fellowships, including a NHMRC C.J. Martin Fellowship, a European Human Frontiers Science Program fellowship and a NHMRC Career Development Award. She has received a number of international, national and local philanthropic grants.

Dr Parish is the author of 18 peer-reviewed publications and has written a number of book chapters and reviews related to stem cells and Parkinson's Disease.

About Professor Colin Pouton

Professor Colin Pouton is Professor of Pharmaceutical Biology, Head of the Department of Pharmaceutical Biology, and Co-Director of Medicinal Chemistry and Drug Action, Monash Institute of Pharmaceutical Sciences (MIPS).

Professor Pouton received his Bachelor of Pharmacy (Honours) from the University of Bath, and his PhD (in Pharmaceutical Science) from the University of London.

He was previously Reader in Pharmaceutical Biology, and Head of the Pharmaceutics Group, Department of Pharmacy and Pharmacology, University of Bath. In 1995, he won the Pfizer Award for Pharmaceutical Science. Professor Pouton served as an elected committee member (1993-2000) and Chairman of the UK Association of Pharmaceutical Scientists (1999-2000), and as an elected member of the committee of the Academy of Pharmaceutical Scientists of Great Britain (2000-2001). Professor Pouton was elected a Fellow of the American Association of Pharmaceutical Scientists in 2003.

Since moving to Monash University, Professor Pouton has established a research group focused on pharmaceutical applications of stem cell biology in collaboration with his MIPS colleague, Dr John Haynes.

Professor Pouton has supervised 38 completed PhD students and is the author of 70 peer-reviewed publications. He has received over $7 million of independent research funds, including a current $1.8 million grant from Pfizer Inc. to investigate low molecular weight inducers of differentiation in mouse embryonic stem cells.

BACKGROUND:

About the Australian Stem Cell Centre

The Australian Stem Cell Centre is Australia's Biotechnology Centre of Excellence. The ASCC was selected in 2002, in a competitive bid process, as Australia's Biotechnology Centre of Excellence (BCE), to capitalise on Australia's significant strengths in the general field of stem cell research. The Centre provides a unique opportunity for stem cell researchers to deliver outcomes that will benefit the wider Australian biotechnology industry and ultimately contribute innovative solutions to human health challenges. The Centre was established with the financial and in-kind support of institutions ("Stakeholders").

The current voting Members, who retain ultimate oversight of the Centre, are: Monash University, University of Queensland, Florey Neuroscience Institutes and University of Adelaide. The additional Stakeholder institutes are: University of Melbourne, Baker IDI, Murdoch Children's Research Institute, Victor Chang Cardiac Research Institute and Mater Medical Research Institute.

The ASCC is governed by a Board of Directors with independent scientific oversight and support from an eminent Scientific Advisory Board.

Total funding of $100 million has been awarded to the ASCC by the Australian Government and is administered by the Australian Research Council and the Department of Innovation, Industry, Science and Research. The funding is provided in instalments from 2002 to 2011. To complement Australian Government funding, the State Government of Victoria's *Science Technology and Innovation* program awarded the Australian Stem Cell Centre a further $11 million to support some key infrastructure in Victoria.

Together the ASCC and partnering organisations support a critical mass of Australian stem cell research that is internationally competitive. The ASCC currently funds research at leading institutes and universities in Victoria, Queensland and New South Wales with the major clusters of activity centred in Victoria and Queensland. By providing stem cell specific funding, the Centre in its first seven years of operation has supported in excess of 200 researchers, more than 70 students in over 30 research projects across Australia.

About Monash University

Monash University is one of Australia's finest universities, and is recognised as a global centre of biomedical and health research. As a member of the 'Group of Eight' universities that between them undertake over 70 per cent of the research conducted in Australia's 39 universities, Monash's capabilities and achievements have resulted in the University gaining a formidable reputation – a remarkable achievement for an institution with a history extending for only 51 years. Over 700 researchers operate within the Faculty of Medicine, Nursing and Health Sciences, not including the significant research contributions made by the Monash Institute for Medical Research, the Australian Regenerative Medicine Institute and a proliferation of research being undertaken in conjunction with our clinical schools and other partner organisations in Australia and worldwide.

A clear mission to improve public health and the human condition on the widest scale permeates both the Faculty's and the University's vision and research objectives.

Monash is also respected for its excellence in healthcare education, consistently ranked in the top 50 universities in the world, and in the top four universities in Australia, by the

Times Higher Education Supplement. Monash consistently receives the highest amount of performance-based funding from the Australian Government of any university in Australia, in recognition of its teaching and learning excellence in health disciplines.

From the time of its formation, Monash University has maintained a strong international focus, and can claim to be Australia's most international university with its own campuses in Malaysia and South Africa, and a centre at Prato in Italy. About 30 per cent of the University's students come from 130 different countries. Together with research collaborations with leading universities all over the world, this results in an international approach that helps to build cultural understanding and mutual respect – essential for peace and security through the challenging times of the 21st century.

About Florey Neuroscience Institutes

Florey Neuroscience Institutes (FNI) is the largest brain research group in the Southern Hemisphere and the sixth largest neuroscience facility in the world. FNI is made up of the Howard Florey Institute, the Brain Research Institute and the National Stroke Research Institute.

With more than 500 research and support staff on two campuses, FNI scientists are searching for cures to the complex, debilitating and sometimes life threatening brain disorders that affect three million Australians.

Through our clinical and basic research facilities in Parkville and Heidelberg, Victoria, we are investigating Parkinson's, Huntington's and Motor Neuron diseases. We are world leaders in Stroke and Epilepsy and brain imaging research and have exceptional teams working on Multiple Sclerosis, addiction and traumatic brain and spinal cord injury among others.