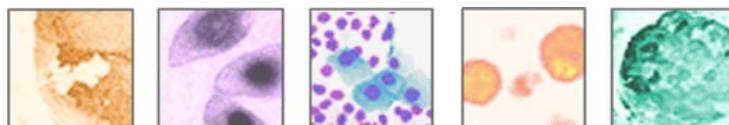


14th Stem Cell Workshop

# Ethics and the Regulations for the Use of Stem Cells

Monday 29<sup>th</sup> Nov, 2010  
10:00am to 3:30pm  
Darlington Centre  
City Road, Camperdown  
University of Sydney



## Welcome to the 14<sup>th</sup> Stem Cell Workshop

In October this year the first clinical trial with human embryonic stem cells (hESCs) commenced in the United States for the treatment of people with paraplegia. This is an exciting milestone in the history of stem cell research and regenerative medicine. It follows on from the discovery in 2007 by a Japanese scientist of induced pluripotent stem cells (iPSCs), which some believe will replace ESCs. Adult stem cells in the forms of mesenchymal precursor cells (MPCs) are also being applied in multiple clinical trials at present; for example, in the treatment heart disease and arthritis. With these exciting events, the need is greater than ever for Australia to continue to re-evaluate what laws should be used to govern the use of stem cells.

Federal parliament is due to announce the creation of a committee to review current laws related to the regulation of stem cells. These laws were created in 2002 and 2005, allowing both the use of human embryonic stem cells (hESC) for medical research and somatic cell nuclear transfer (SCNT).

The current workshop is designed to cover the ethics and regulations of the use of stem cells in Australia, and to provide an opportunity for those involved to understand the points of view of the many different stakeholders, including government, regulatory bodies, the Australian Stem Cell Centre, universities, end users, the media and private companies. We are lucky to have with us today representatives to speak from all of these perspectives.

We hope that by the end of the workshop you will have clearer understanding of the status of stem cell research in Australia and how we are progressing in this important arena.

Kind regards,



**Carrie Hardie**  
Manager



**Dr Bernie Tuch**  
Director

**NSW Stem Cell Network**

# ETHICS AND THE REGULATIONS FOR THE USE OF STEM CELLS

9:00am Registration opens / coffee

10:00am Welcome and Opening Remarks  
**Professor Ian Kerridge**

## Session 1: Stem cells in the clinic

Chair: **Professor Ian Kerridge, Centre for Values, Ethics, and Law in Medicine**

10:15am Stem cells: What they are, where they come from, what they do, and where they could end up  
**Dr. Bernie Tuch**

10:40am Clinical development of Mesenchymal Precursor Cells: Regulatory requirements and expectations  
**Dr. Kilian Kelly, Mesoblast Ltd**

11:05am Media experience with stem cells  
**Ms. Julie Robotham, Sydney Morning Herald**

11:25am Community awareness and acceptance of stem cells: Is there an expectations vacuum?  
**Dr. Megan Munsie, Australian Stem Cell Centre**

11:50am Patentability of stem cell technologies  
**Dr. Daniel Schaft, Shelston IP**

12:15pm Cow-boys, mavericks and trailblazers: Stem cell researchers' ambivalence about their field  
**Dr. Nicola Marks, University of Wollongong**

12.40pm Lunch / networking

## Session 2: Regulation and approvals of stem cell therapies

Chair: **Joanna Knott, Australasian Spinal Research Trust**

1:25pm Regulation of cell therapies in Australia  
**Dr. Ian Prosser, Therapeutic Goods Administration**

1:50pm Regulating human stem cell research in Australia: Issues for the Australian federal review in 2010  
**Professor Loane Skene, Australian Health Ethics Committee**

2:15pm Future of stem cell research  
**Ms. Kerry Doyle, NSW Office of Science and Medical Research**

2:40pm Australian embryo research regulation: The light and the dark side  
**Dr. Julia Schaft, Sydney IVF Stem Cells**

3:05pm Refreshments / networking

## **STEM CELLS: WHAT THEY ARE, WHERE THEY COME FROM, WHAT THEY DO, AND WHERE THEY COULD END UP**

It has been 12 years since the first human embryonic stem cell (hESC) line was derived from a spare fertilized egg in an IVF clinic. Last month the first clinical trial with neuronal precursor cells derived from these pluripotent cells for the treatment of paraplegia commenced, with approval of the FDA in the USA. A second trial, for treatment of blindness in the young, is foreshadowed shortly, with yet another trial, for treatment of Type 1 Diabetes, further distant.

Three years ago, a Japanese professor showed the world that it is possible to produce pluripotent stem cells, without the need to use embryos. This requires the insertion of several genes or proteins to de-differentiate an adult cell, for example, one from the skin. Whilst the risk of using such induced pluripotent stem cells clinically is greater than that of a hESC, they are revolutionizing the stem cell arena. Like the hESC, they are being used to understand disease processes, for drug development, monitoring development of genetic disorders, and assisting in developing therapies for cancer.

The multipotent stem cell is derived from adult cells, cord blood or fetal tissue. Such cells derived from the bone marrow have been used with great success for 50 years in the treatment of haematological malignancies such as leukaemia. Cord blood has been used clinically for a similar purpose for two decades now, but mesenchymal stem cells (MSC), derived from bone marrow, peripheral blood or placenta only very recently and for different purposes. Trials underway at present with MSC include heart disease, arthritis, and diabetes. Although the donor and the recipient are the same in many trials, in some this is not so. In these cases, it is the capacity of the stem cell to evade immune detection which is being relied upon to prevent their rejection by the recipient.

We live in exciting times, with stem cells and regenerative medicine being a key new technology, rivaling genetic engineering and climate change for our attention.



### **Dr Bernie Tuch**

**Director, NSW Stem Cell Network; Director, Australian Diabetes Therapy Project, CSIRO**

Dr Bernie Tuch is a translational clinician, who not only treats patients with endocrinological disorders but has a passion for carrying out cutting-edge research with stem cells. In the 1980s he and his colleagues pioneered the transplantation of human fetal tissue as a possible therapy for Type 1 Diabetes. In 2006, Dr Tuch led an innovative clinical trial with insulin-producing cells from organ donors, with the cells being placed in microcapsules made from seaweed to overcome the need for anti-rejection drugs. Most recently, he left his position as Professor of Medicine at the University of New South Wales, and joined the CSIRO to direct the Australian Diabetes Therapy Project. There he aims to take encapsulated stem cells to the clinic as a therapy for Type 1 Diabetes, thereby overcoming the need for insulin. He has been the Director of the NSW Stem Cell Network since its inception in 2002.

## **CLINICAL DEVELOPMENTS OF MESENCHYMAL PRECURSOR CELLS: REGULATORY REQUIREMENTS AND EXPECTATIONS**

Mesoblast is developing Mesenchymal Precursor Cell (MPC) technology for a wide range of therapeutic indications, with a number of clinical trials underway in Australia and the USA, and additional studies planned to commence in other countries in the near future. MPCs are isolated from adult human bone marrow using monoclonal antibodies, in a proprietary manufacturing process based on technology originally developed at Hanson Institute/IMVS in Adelaide. A key feature of MPCs is the absence of immune response when cells from a donor are administered to unrelated recipients, which facilitates use in an allogeneic or “off the shelf” manner.

The major challenge in navigating the regulatory pathway for MPCs, or indeed any cell-based therapy, is the lack of clarity about regulatory requirements for this class of product. There is also limited consistency between different regulatory jurisdictions.

The forthcoming new “biologicals framework” regulations in Australia should bring clarity and a set of regulations specifically tailored to this type of therapeutic product for the local market. However, at this time some questions remain, in particular with regard to the regulatory approval requirements for clinical trials, and the type and size of clinical trials required to support entry of different products onto the Australian Register of Therapeutic Goods (ARTG). It is also unlikely that discrepancies in regulatory requirements between different markets will be eliminated in the near future.

Nonetheless, Mesoblast has been successful thus far in gaining approval to progress with our clinical trial programmes in both Australia and the USA. Furthermore, Mesoblast has been granted a TGA manufacturing authorisation. Our approach, wherever possible, is to conduct research in accordance with requirements in multiple jurisdictions. Where it has not been possible and/or logical to conduct studies in line with the existing standard pharmaceutical guidance, alternative strategies have been proposed and justified.



**Dr Kilian Kelly**  
**Director Regulatory and Clinical, Mesoblast Ltd**

Kilian has worked in the biotechnology/pharmaceutical development field for over 10 years. His experience encompasses all major global markets, numerous therapeutic areas and a diverse range of products, including cellular therapies, monoclonal antibodies and small molecules. Kilian joined Mesoblast in November 2009 to lead the clinical and regulatory team in the Melbourne office, which is developing Mesenchymal Precursor Cell (adult stem cell) technology for a range of indications.

Before joining Mesoblast, Kilian was a Project Leader at Kendle International’s Regulatory, Development and Commercialisation group, where he worked with a variety of clients, from start-up biotechnology companies to Big Pharma. He previously worked in regulatory affairs roles in the UK with Amgen and AstraZeneca, and prior to that conducted pharmaceutical clinical trials in an academic setting. Kilian is a registered pharmacist and also has prior experience practicing in community and hospital pharmacy.

## MEDIA EXPERIENCE WITH STEM CELLS

Stakeholders generally agree that informed community input is essential to the creation of workable regulation for the use of stem cells, just as it is to other controversial techniques such as assisted reproductive technologies and xenotransplantation. Most ordinary people form their views of such issues at least partly in response to mass media coverage, but many factors conspire to make good quality coverage difficult to achieve: poor scientific literacy in the community, polarised scientific and ethical perspectives among experts, the abstract and contingent nature of any future benefits, and the demands of an accelerating news cycle. The presentation will detail these issues, with examples, to suggest how scientists and ethicists can constructively engage the public through their work with journalists.



**Ms Julie Robotham**  
**Health Editor, *Sydney Morning Herald***

Julie Robotham is the Health Editor at the Sydney Morning Herald, where she leads health and medical news coverage across the newspaper. In 2007-8 she was a Knight Science Journalism Fellow, based at Massachusetts Institute of Technology. She was 2009 winner of the Royal College of Pathologists of Australasia Media Award, for a series of articles about gene patenting, and the print category winner in 2010 of the Clinical Oncological Society of Australia's Luminous Media Award, for an article about the commercial interests behind a new form of prostate cancer surgery.

## COMMUNITY AWARENESS AND ACCEPTANCE OF STEM CELLS: IS THERE AN EXPECTATIONS VACUUM?

Around the globe stem cell science has captured the public's imagination like almost no other scientific field in recent times. Every breakthrough attracts immediate media interest, further heightening the community's expectation of new therapies to treat incurable diseases and illness. Recent survey results showed that the use of stem cells remains one of the most accepted biotechnology applications in Australia, with the highest levels of awareness, but lowest perceived risk. While the continuing community support for stem cell research is encouraging, there is a danger that community expectations are outpacing the rate of scientific and medical progress in the stem cell field and that this is creating an "expectations vacuum" with the Australian public. Such a vacuum creates the opportunity for exploitation of the Australian public by overseas companies and clinics offering stem cell treatments now with little or no scientific basis let alone proof of safety. Whilst the progress of medical research can often seem painfully slow and frustrating for those in need, we need to temper the excitement associated with each development in the field with a realistic perspective on when new stem cell treatments will and should be available in Australia and elsewhere. This presentation will provide an overview of the educational strategy that the Australian Stem Cell Centre (ASCC) has adopted to address this issue.

### **Dr Megan Munsie**

#### **Senior Manager - Research & Government Australian Stem Cell Centre**



Dr Megan Munsie's career in stem cell research spans her proof-of-concept somatic cell nuclear transfer studies in mouse and her involvement in deriving one of Australia's first HESC lines; through to her participation in the reform of Australian legislation governing this area and her participation on the ISSCR Task Force on the Clinical Translation of Stem Cells.

Megan is currently a member of the Australian Stem Cell Centre's management team where she is responsible for managing its diverse research portfolio, its government interactions and the production of educational material and initiatives.

She is a member of advisory committees to the Victorian Government Assisted Reproductive Treatment Authority and the Australia New Zealand Spinal Cord Injury Network, a member of the Government Affairs, Ethics and Public Policy Committee of the Australasian Society for Stem Cell Research and regularly provides talks to numerous secondary school, community and patient advocacy group meetings on stem cell science. Megan received her undergraduate degree from QUT and a Masters and a Doctorate of Philosophy from Monash University. In addition to her work in the stem cell field, Megan has over 12 years' experience working as a clinical embryologist in IVF practices around Australia.

## PATENTABILITY OF STEM CELL TECHNOLOGIES

In Australia, the patentability of stem cell-related inventions has been considered by the Patent Office in two pivotal cases. The dividing lines between patentable and non-patentable inventions provided by the Patent Office draw interesting distinctions between various aspects of stem cell research. Similar issues are currently being considered by the courts in a number of other jurisdictions further illustrating the technical as well as the ethical complexities of patenting stem cell technologies. Daniel Schaft has extensive research experience in the use of stem cell technology and will illustrate some of the difficulties in assessing whether an invention relating to stem cells can be considered patentable under Australian law.

### **Dr. Daniel Schaft** **Shelston IP**



Daniel is a patent attorney at Shelston IP and practises in the fields of biotechnology, stem cell biology, pharmaceuticals, molecular and developmental biology. He holds a joint doctorate degree (Dr. rer. nat. / PhD) from the University of Giessen and the European Molecular Biology Laboratory (EMBL) Heidelberg, Germany and a Master of Industrial Property (MIP) from the University of Technology Sydney.

During his PhD Daniel developed biochemical and proteomic techniques to characterize chromatin modifying protein complexes. Upon completion of his PhD, he worked as a Postdoctoral fellow at the University of Technical Sciences in Dresden, Germany and, in Australia as a Fellow of the Human Frontiers Science Program at the Victor Chang Cardiac Research Institute in Sydney. His research has focused on mechanisms of chromatin regulation and transcriptional control underlying the development of congenital heart disease.

Daniel is a member of the NSW Stem Cell Network, the Australia and New Zealand Society for Cell and Developmental Biology (ANZSCDB), the Intellectual Property Society of Australia and New Zealand (IPSANZ) and the Institute of Patent and Trade Mark Attorneys of Australia (IPTA).

## **COW-BOYS, MAVERICKS AND TRAILBLAZERS: STEM CELL RESEARCHERS' AMBIVALENCE ABOUT THEIR FIELD**

Science is a central part of contemporary society, but does not automatically command public trust. A number of reasons have been given for this, including the public's difficulty in grasping the promises and pitfalls of future applications, but also scientists' difficulty in conveying these. In particular the latter have been accused of placing too much emphasis on the exciting prospects and not reflecting enough on the risks. Here, drawing on interview data with 54 stem cell researchers from the UK and Australia, the ways in which these scientists talk about their work is examined. They raise important concerns with regards to the internationalisation of science, clinical trials, informed consent and commercialisation. The analysis of these discourses show that these scientists reflect on the unintended consequences of their research. This may offer a chance of improving trust between science and members of the public.



**Dr. Nicola Marks**  
**University of Wollongong**

Nicola completed an undergraduate degree in Natural Sciences at Cambridge University, specialising in genetics. She then worked in genetic diagnostics and research in Australia for a year before going to Edinburgh to start a 4 year Master/PhD at the Medical Research Council Human Genetics Unit. However, she soon realised that she was more interested in the broader societal issues about science and ended up completing her PhD on the topic of stem cell research and public engagement. She then worked as a post-doc in Edinburgh for one year on the same topic, organising events where scientists and various members of the public came together.

In 2009, she moved to Wollongong to take up a lectureship in Science and Technology Studies. Her research and teaching interests include science-public interactions, cutting edge science (especially stem cell research and reproductive technologies) and the social and ethical issues surrounding science in general.

# REGULATION OF CELL THERAPIES IN AUSTRALIA

ABSTRACT UNAVAILABLE AT TIME OF PRINTING

## **Dr. Ian Prosser**

**Senior Medical Adviser, Biological Science Office of Scientific Evaluation, Therapeutic Goods Administration**

Dr Ian Prosser is Senior Medical Adviser to the Biological Sciences Section, Office of Scientific Evaluation, at the Therapeutic Goods Administration, which is responsible for regulation of cell and tissue therapies in Australia, including blood and blood components. He also works as a clinical Haematologist in private practice in Canberra. He has interests in transfusion medicine, laboratory and clinical haematology, clinical research and post-graduate medical education.

## REGULATING HUMAN STEM CELL RESEARCH IN AUSTRALIA: ISSUES FOR THE AUSTRALIAN FEDERAL REVIEW IN 2010

This year, the Australian legislation on human cloning and stem cell research is due to be reviewed by a federal committee (yet to be appointed). The terms of reference for the review are set out in the legislation that implemented the Lockhart Committee's recommendations in 2006.\*\* The matters to be reviewed include developments in assisted reproductive technology and embryonic stem cell research; actual or potential applications of such research; community standards; the effectiveness of the legislation, NHMRC guidelines and the licensing system; and any research or clinical practice which has been prevented by the legislation.

Developments since 2006 include the derivation of induced pluripotent stem (iPS) cells from somatic cells; the differentiation of iPS cells into other types of cells, in animal and human models; the creation of cybrids (human-animal embryos) in the UK to obtain human stem cells for research; 'egg sharing' arrangements in the UK to increase egg donation for research; the authorisation of public funds in New York to compensate women for donating eggs; the first steps to derive human gametes from skin cells in Newcastle UK, et al; and a variety of clinical trials to establish the safety and efficacy of various stem cell treatments.

The reported clinical trials and clinical applications may be changing community views. Activities that are widely condemned or prohibited are likely to remain prohibited, like developing a human embryo for more than 14 days; implanting a human-animal embryo, or an embryo used in research, in a woman's body; and implanting a human embryo in an animal or vice versa. However, there may be increased support for allowing women to be paid for donating eggs and the creation of cybrids to derive human stem cells for research – both of which are currently prohibited in Australia.

\* In 2005, Professor Skene was Deputy Chair of the Lockhart Committee on Human Cloning and Embryo Research and became principal spokesperson for the Committee after the sudden death of the Chair, the late Justice Lockhart AO, in January 2006.

\*\* Prohibition of Human Cloning for Reproduction Act 2002 (Cth), s 25A; Research Involving Human Embryos Act 2002 (Cth), s 47A.

### **Professor Loane Skene** **Australian Health Ethics Committee and Melbourne Law School, The University of Melbourne**



Professor Loane Skene LL.D (UMelb), LL.M (Mon), LL.B (Hons) (UMelb), is a Professor of Law in the Faculty of Law and an Adjunct Professor in the Faculty of Medicine Dentistry and Health Sciences at the University of Melbourne. She is a member of the Australian Health Ethics Committee, one of the principal Committees of the National Health and Medical Society; and a Deputy Director of the Centre for Law and Human Genetics at the University of Tasmania. She has served on numerous federal and state advisory committees, especially in relation to genetics and the law.

In 2005, she was Deputy Chair of the Lockhart Committee on Human Cloning and Embryo Research and became principal spokesperson for the Committee after the sudden death of the Chair, the late Justice Lockhart AO, in January 2006.

She is the author of two books on medical law, (including the widely used text, *Law and Medical Practice*, 3<sup>rd</sup> ed, LexisNexis Sydney, 2008); and numerous chapters in books and articles in Australian and overseas legal, medical and scientific journals. In 2003, she was awarded a Centenary Medal for 'Service to Australian Society through the Exploration of Legal and Ethical Issues of Health Care' and in 2007 she was named by the Australian Financial Review among Australia's most powerful cultural figures.

## THE FUTURE OF STEM CELL RESEARCH

Stem cells hold great promise in a wide range of scientific endeavours, from the development of therapeutics to understanding human development. However, the future of stem cell research in Australia will be dictated not only by scientific capacity and discovery but by the level of community understanding of the science. The legislative framework for this field has been a dynamic one for this reason.

### **Ms. Kerry Doyle**

#### **Executive Director, Innovation, Science and Industry Analysis, State and Regional Development and Tourism Industry & Investment**



Kerry Doyle has been employed in the NSW Public Sector, since 1994 and has undertaken a variety of senior roles in the NSW Cabinet Office, the NSW Maritime Authority, the Department of State and Regional Development and Industry and Investment NSW.

Kerry is currently the Executive Director of Innovation, Science and Industry Analysis in Industry and Investment NSW. Her responsibilities include working with the NSW Innovation Council and the NSW Chief Scientist and Scientific Engineer, management of the Office for Science and Medical Research and development of business and industry strategy.

Kerry's experience within Government has included roll out of the Government's biotechnology strategy, development of policy and funding programs for science and medical research, development of the State's innovation agenda, and work on policy matters including education, health, tax reform, environmental reform and state development.

She has also been involved in highly sensitive matters such as legislation regulating the use of human embryos and gene technology, achieving successful outcomes and gaining support from the research, industry and broader community.

In 2005, Kerry received an Australia Day Award for public service in the areas of science and medical research.

## **AUSTRALIAN EMBRYO RESEARCH REGULATION: THE LIGHT AND THE DARK SIDE**

Continued investment in research and a strong belief in the benefits of human embryonic stem cell technology have contributed to Sydney IVF's unique position as a world class Australian ART clinic.

Since its inception in 1986, Sydney IVF has made many contributions not only to advanced IVF technologies but is also one of the most active research facility involved in human embryo and stem cell research. It is holding 8 out of 9 embryo research licences in Australia. These achievements would not have been possible without the permissive legislative framework in Australia with regards to human embryo research, but it also comes at a price: compliance with the Embryo Research Act and its administering body, the NHMRC Licensing Committee in Australia is weighing down heavily on research institutions and is slowing or at times even inhibiting scientific progress due to its exorbitant maintenance costs.

How much regulative pressure is appropriate and when does it start becoming inhibitive? How much regulation do we really need? Different legislative models and alternatives in various countries will be discussed.



### **Dr. Julia Schaft**

#### **Manager for Strategy and Regulations, Sydney IVF Stem Cells**

Dr Julia Schaft, has a background in developmental and molecular biology. She obtained her PhD at the University of Giessen and the European Molecular Biology Laboratory (EMBL) and has gained 6 years of experience in animal stem cell techniques, including in vitro differentiation and genetic manipulation as well as transgenic mouse production.

After joining Sydney IVF in 2004, Dr Schaft was trained in all aspects of human embryonic stem cell derivation and culturing techniques. While using her extensive professional contacts in Europe and the U.S. to establish communications with potential pharmaceutical and related industry customers, Dr. Schaft has played a key role in securing funding and other opportunities furthering Sydney IVF's stem cell commercialization strategy. Since 2008 she has been Scientific and Regulatory Project Manager for the Somatic Cell Nuclear Transfer (SCNT) Project at Sydney IVF and has recently (February 2010) taken up a new role as Manager of Strategy and Regulation for the Sydney IVF Biotech division.

Apart from supervision and management of all eight of Sydney IVF's embryo research licences, Dr Schaft is identifying and facilitating strategic alliances with international commercial and academic partners to further disease research and drug development driven by diseases-specific stem cell technologies.

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## NSW Stem Cell Network

Contact: Carrie Hardie  
Tel: +61 (0) 2 9552 9981  
Email: [c.hardie@stemcellnetwork.org.au](mailto:c.hardie@stemcellnetwork.org.au)  
URL: [www.stemcellnetwork.org.au](http://www.stemcellnetwork.org.au)