NSW Stem Cell Network

10th Stem Cell Workshop

"Novel Clinical Trials with Stem Cells"

Friday 26, October 2007
12:30 pm to 5:30 pm
John Dwyer Lecture Theatre
Edmund Blacket Building
Prince of Wales Hospital
Randwick, NSW

Welcome to the 10th Stem Cell Workshop

The motivation behind the theme of this workshop, "Novel Clinical Trials with Stem Cells," is the desire to explore the extent to which the promise of stem cell research has delivered. We are sure you've been wondering, as we have, how far the field has come, and what the clinical applications of stem cells look like, as we take stock at the end of 2007.

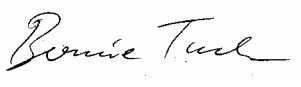
This Workshop provides a snapshot of the scene in Australia which includes an important network of international collaborations in the commercial and public realms. The story that's ready for the telling shows the dominance of adult stem cells. Mesoblast Ltd. with its sister company Angioblast Systems Inc. has undertaken progressive clinical studies in fracture repair, spinal fusion and heart disease. Ian Lewis, from the Royal Adelaide Hospital, talks about their participation in an international multi-centre study to evaluate the effectiveness of mesenchymal stem cell infusion in severe acute graft versus host disease. John Moore, from St. Vincent's Hospital, presents data on the Australian experience of autoimmune diseases and discusses future trends. Janet Macpherson from Johnson & Johnson Research Pty. Ltd. talks about their clinical work on haematopoietic stem cells as gene delivery agents for HIV. That completes session one of the Workshop.

Pre-clinical studies from the Victor Chang Cardiac Research Institute suggest granulocyte-colony stimulating factor (G-CSF) holds promise for mobilising stem cells to treat ischemic heart disease, and in session two Peter MacDonald presents the outcome of studies assessing the clinical safety and efficacy of this therapy. Chris Juttner, a consultant for the California company StemCells Inc., presents the preclinical and initial clinical experiences of StemCells Inc. with human neural stem cells. Kerry Atkinson of the Mater Hospital and Mater Medical Research Institute discusses preclinical and clinical application of mesenchymal stem cells. The Workshop closes with a discussion by Albert Farrugia from the Therapeutic Goods Administration on the proposed changes to clinical trial regulation in Australia.

Warm thanks go to all those who have supported the organisation of the 10th Stem Cell Workshop. Special thanks go to Kelvin Hopper, Executive Chairman of Innovation Dynamics, for opening the Workshop, and to our two chairs, Perminder Sachdev from the Neuropsychiatry Unit at the Prince of Wales Hospital, and Phil Waite from the Neural Injury Research Unit at the University of NSW, for sharing their time and expertise.

Enjoy this Workshop and we look forward to keeping in touch through the Network.

Kind regards,



Nola Camden ***Prof Bernie Tuch***
Manager ***Director***

NSW Stem Cell Network

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10th Stem Cell Workshop

Novel Clinical Trials with Stem Cells

Presented by the NSW Stem Cell Network

12:30pm	Registration
1:00pm	Welcome **Dr Kelvin Hopper, Executive Chairman of Innovation Dynamics**
Session 1: Chair:	**STEM CELLS FROM BONE MARROW AND PERIPHERAL BLOOD** Professor Perminder Sachdev, Neuropsychiatric Unit, Prince of Wales Hospital
1:10pm	Mesenchymal precursor cell clinical studies in tissue repair and regeneration **Dr Donna Skerrett, Mesoblast Ltd. (LAST MINUTE CANCELLATION)**
1:35pm	Mesenchymal stem cells to treat acute graft versus host disease **Dr Ian Lewis, Hanson Institute, University of Adelaide**
2:00pm	Hematopoietic stem cell transplantation for severe auto-immune diseases **Dr John Moore, St Vincent's Hospital, Sydney**
2:25pm	Hematopoietic stem cells as gene delivery agents for HIV **Dr Janet Macpherson, Johnson & Johnson Research Ltd.**
2:50pm	Afternoon Tea
Session 2: Chair:	**STEM CELLS FROM OTHER SOURCES** Professor Phil Waite, Neural Injury Research Unit, University of New South Wales
3:20pm	Mobilisation of autologous stem cells with GCSF - a treatment strategy for angina? **Prof Peter MacDonald, Victor Chang Cardiac Research Institute**
3:45pm	Human neural stem cells: preclinical and initial clinical experience **Dr Chris Juttner, Consultant for StemCells Inc.**
4:10pm	Preclinical and clinical studies with mesenchymal stem cells **Prof Kerry Atkinson, Mater Medical Research Institute**
4.35pm	Regulation of therapeutic goods derived from stem cells **Prof Albert Farrugia, Therapeutic Goods Administration**
5:00pm	Refreshments

MESENCHYMAL PRECURSOR CELL CLINICAL STUDIES IN TISSUE REPAIR AND REGENERATION (LAST MINUTE CANCELLATION)

Cell therapy for tissue repair has been explored with somatic cells, hematopoietic progenitor cells, and mesenchymal cells. Autologous or allogeneic cells derived from bone marrow, peripheral blood, bone, muscle or fat may affect local signals mediating endogenous stem cell recruitment, factor release, signal transduction, and cell differentiation and regeneration.

Mesoblast's mesenchymal precursor cells are identified by expression of stromal markers and have demonstrated capacity for pericyte stabilization, arteriogenesis, osteogenesis, growth factor secretion, and potent immunomodulatory effects. The secretion of immunoregulatory growth factors, such as TGF-beta and IL-6 may enhance functional repair by abrogating inflammatory responses that impair endogenous tissue repair and may also facilitate function and engraftment of allogeneic source cells in the host.

Mesoblast Ltd. and Angioblast Systems, Inc. allogeneic, mesenchymal precursor cells are derived from human, adult bone marrow. The Stro-1 population is immuno-selected and ex-vivo expanded to provide a characterized, 1000-fold purer product with documented potency. Manufacturing is performed in a c-GMP facility. Cryopreserved cells express Stro-1, Stro-3, and are negative for hematopoietic markers such as CD45 and CD34. The cells differentiate to ostegenic, adipogenic, and chondrogenic cells in tissue specific culture assays.

We studied MPCs in allogeneic, ovine models of fracture repair, spinal fusion, meniscal injury, and acute and chronic myocardial ischemia. Functional endpoints and short and long term safety endpoints were assessed. Allogeneic, mesenchymal precursor cells were well tolerated at all doses studied. All cells were delivered locally to the tissue repair site. There were no episodes of remote migration, cytogenetic instability, tumorigenicity, or cell mediated inflammatory responses at the short or long term endpoints. Sustained functional improvement was demonstrated in acute and chronic cardiac models. Bone regeneration and synovial repair were noted in the fracture and menisical injury models, respectively and exceeded that obtained when compared with current therapy controls.

Pilot clinical studies assessed the feasibility of delivery systems and carriers, the manufacturing process, and study procedures. Subsequent clinical studies in fracture repair, spinal fusion, and ischemic heart disease will be presented.

Donna L. Skerrett, MD, MS

Medical Director, Mesoblast Ltd., Melbourne

Donna L. Skerrett, MD, MS represents Mesoblast Ltd, an adult stem cell company based in Melbourne, Victoria. Mesoblast's unique, proprietary platform utilizes immuno-selection of allogeneic, immune tolerant, mesenchymal precursor cells prior to ex-vivo expansion and cryopreservation. Mesoblast's allogeneic cells, Revascor ™ and NeoFuse ™ are in clinical studies in the US and trials will soon commence in

Australia. Dr. Skerrett comes to Mesoblast from New York, NY where she holds an academic position at Weill Cornell Medical College as Director of Transfusion Medicine and Cellular Therapy. Dr Skerrett received her undergraduate degree from the University of Pennsylvania, her medical degree from Temple University (both in Phila, PA, USA), and a master's degree in biostatistics for clinical research methods from Columbia University in New York, NY.

Following many years of experience in conventional transfusion medicine and stem cell therapy, Dr Skerrett initiated pilot studies in non-homologous and non-hematopoietic stem cell therapies. These studies included 1) autologous, mobilised blood stem cells in ischemic cardiac disease, 2) autologous bone marrow cells in LVAD bridge-to-transplant patients, 3) autologous, mesenchymal precursor cell therapy in ischemic heart disease, 4) autologous, mesenchymal precursor cell therapy in fracture repair, 5) the first in human experiences using Mesoblast's allogeneic, immuno-selected mesenchymal precursor cells in acute ischemic heart disease, and 6) spinal fusion. Translation from research concept to the human clinic required the design and interpretation of relevant large animal studies, development of large scale clinical grade manufacturing procedures, clinical trial design, and successful regulatory filings with the US FDA to initiate phase 2a studies in the US and Australia.

MESENCHYMAL STEM CELLS TO TREAT ACUTE GRAFT VERSUS HOST DISEASE

In addition to haemopoietic stem cells, bone marrow (BM) also contains Mesenchymal Stem Cells (MSC), which can differentiate into multiple mesodermal lineages including bone, cartilage, muscle and fat. In the laboratory these cells are isolated from the adherent layer of BM and have the appearance of fibroblast like cells which can be induced to differentiate under appropriate conditions to bone, cartilage, muscle and other tissues. This property has led to the suggestion that MSC may have a role in tissue repair. In addition to the potential role in tissue repair and regeneration, MSC have also been shown to have unique immunomodulatory properties. They are immunogenic, meaning they do not elicit an immune response when injected into an allogeneic recipient and are not rejected. MSC also have potent immunosuppressive properties. This property has been exploited in the treatment of severe acute graft versus host disease (aGVHD), a life threatening immunological complication of allogeneic bone marrow transplantation, with encouraging preliminary results. At the Royal Adelaide Hospital we are participating in an international multi-centre study to evaluate the effectiveness of MSC infusion in severe aGVHD. To date we have treated three patients with encouraging responses seen in two of these. Together with the results from overseas institutions MSC infusion appears to be an encouraging modality in the treatment of severe aGVHD which has previously been associated with high mortality rates.

Ian Lewis MB BS, PhD, FRACP, FRCPA

Senior Consultant Haematologist, Institute of Medical and Veterinary Science (IMVS) and Royal Adelaide Hospital (RAH)

Ian Lewis is a Senior Consultant Haematologist at the Institute of Medical and Veterinary Science (IMVS) and Royal Adelaide Hospital (RAH), Adelaide, Australia. He is Head of the Therapeutics Products Facility at the IMVS which is a suite of three clean rooms designed for the manufacture of cellular products under the requirements of Good Manufacturing Practice. Dr Lewis trained in haematology at the RAH and IMVS and undertook post-doctoral studies at the University of Minnesota, USA. His clinical interests include the treatment of leukaemia and bone marrow transplantation with research interests in umbilical cord blood biology, use of mesenchymal stem cells and development of cellular therapies.

HAEMOPOIETIC STEM CELL TRANSPLANTATION (HSCT) FOR SEVERE AUTO-IMMUNE DISEASES

HSCT for autoimmune diseases became a reality in the human setting after extensive evidence from animal models suggested a role for the procedure using not only allogeneic grafts but also autologous grafts. Further evidence to support the use of HSCT for autoimmune diseases was provided by the publication of case reports of patients who received both allogeneic and autologous bone marrow transplants for co-existant malignancies or aplastic anaemia. Given the increasing safety of HSCT with the advent of peripheral blood stem cells it was not unreasonable to commence pilot studies of autologous HSCT for patients with these diseases who experience significant morbidity and mortality. Over 400 patients have now been treated using HSCT for a range of diseases including rheumatoid arthritis, scleroderma, multiple sclerosis and SLE. Data has now accumulated in Phase I/II trials to allow design of randomized trials under the auspices of the European Bone Marrow Transplantation (EBMT) group. These trials may more fully elucidate the role of this procedure in these patients.

Mechanisms of response and recurrence in HSCT for autoimmune diseases are not clearly apparent. Debate continues concerning the role of tolerance and immune re-setting or myeloblation in these diseases and some data on the Australian experience in autoimmune diseases will be presented. The use of HSCT for autoimmune diseases remains an area of continued activity and research in transplant units around the world and some future trends are discussed.

John Moore, MB BS, MD, FRACP, FRCPA

Haematology Department, St. Vincent's Hospital, Sydney

John Moore graduated from Sydney University in 1989 and completed his residency and Haematology Advanced training at Royal North Shore Hospital. He was awarded Fellowship of the College of Physicians and Pathologists in 1997. He continued his training at the Westmead Children's Hospital and subsequently became the Bone Marrow Transplant Coordinator at the Royal Free Hospital, London from 1997-1998. He returned to Australia in 1998 and completed a Doctorate of Medicine at St. Vincent's Hospital whilst researching the role of haemopoietic stem cell Transplantation for Severe Rheumatoid Arthritis. He is currently a Staff Specialist in the Haematology Department at St. Vincent's Hospital and a Visiting Medical Officer at the Mater Hospital and St. Vincent's Private Hospital. He was the Chair of the Bone Marrow Transplant Committee of the Australian Leukaemia and Lymphoma Group (ALLG) from 2004-2006. He has been a member of the Executive of the ALLG from 2002-2006, Secretary from 2002-2004 and on the Safety and Data Monitoring Board from 2002-2006. He has been on the Medical Advisory Committee of the Leukaemia Foundation from 2002-2004. Dr Moore has been involved in clinical trials for the past ten years and is presently a Chief Investigator at St. Vincent's Hospital in 8 different clinical trials. His research interests include thymic and endothelial differentiation of haemopoietic stem cells. He is a conjoint Senior Lecturer in Medicine at the University of New South Wales.

HEMATOPOIETIC STEM CELLS AS GENE DELIVERY AGENTS FOR HIV

Human immunodeficiency virus type 1 (HIV-1) is the primary etiologic agent for Aquired Immune Deficiency Syndrome (AIDS). HIV-1 is a complex RNA virus that integrates into the genome of host cells and replicates intracellularly. Ribozymes are catalytic RNA molecules with enzyme-like cleavage properties, that can be designed to target specific RNA sequences within the HIV-1 genome. In addition to the genomic RNA, several RNA intermediates, including splice variants, can be targeted by a single ribozyme. Ribozyme gene therapy for HIV-1 infection is a therapeutic approach that offers several advantages over conventional therapies in that it can potentially impact on both viral load and restoration of the immune system, particularly if the ribozyme is introduced in hematopoietic stem cells capable of populating the lympho-hematopoietic system with protected progeny cells. HIV gene therapy may effect viral suppression and facilitate immune restoration without problems of patient compliance. The anti-HIV-1 ribozyme has been tested in two separate Phase I Clinical Trials, and a large multi-centre Phase II clinical trial is currently in progress.

Dr Janet Macpherson

Johnson & Johnson Research Pty. Ltd., Sydney

Janet Macpherson, PhD is Research Manager and Global Product Scientist, HIV Gene Therapy at Johnson & Johnson Research Pty. Ltd. (JJR), Sydney, Australia. After obtaining a BAppSci (Med Tech) degree from Royal Melbourne Institute of Technology, Janet commenced her research career under the mentorship of Prof John Martin (Sheffield) and Prof David Penington (AC) studying platelets in health and disease. In Sydney, Janet studied the role of lipid-derived inflammatory mediators in gut mucosa, and became interested in Platelet Activating Factor, completing a PhD at St George Hospital on the hematopoietic ontogeny of human mast cells. In 1993 Janet took up a Postdoctoral position with Geoff Symonds within the antiviral team at Johnson & Johnson Research, to advance the potential use of gene suppression technologies as antiviral agents. Janet has moved from basic research to clinical development, involved since inception of the clinical development program through the JJR-sponsored Phase I and II trials conducted in Australia and the USA. Her skills include all laboratory aspects of the cell-delivered HIV gene therapy clinical trial program as well as knowledge of regulatory agency requirements for advanced cell therapeutics. Most recently, Janet has managed the viral vector and cell manufacturing aspects of the current multi-centre Phase II trial, both in Australia and in the USA.

Janet is a member of numerous professional societies including the International Society of Cellular Therapies, American Society of Gene Therapy, International Society of Experimental Haematology, and has served on several sub-committees. She is an inventor on patents in the field of cell-delivered gene therapy, and continues to be involved in gene-modified cell manufacturing development.

MOBILISATION OF AUTOLOGOUS STEM CELLS WITH GCSF – A TREATMENT STRATEGY FOR ANGINA?

Pre-clinical studies suggest granulocyte-colony stimulating factor (G-CSF) holds promise for treating ischemic heart disease (IHD), however its clinical safety and efficacy in this setting remains unclear. Therefore, we elected to evaluate the safety and efficacy of G-CSF administration in patients with refractory 'no-option' IHD. In our initial study (GAIN-1), the safety and efficacy of G-CSF administration and also, an intracoronary infusion of G-CSF-mobilized CD133+ cells were assessed in 20 patients (18 males, 2 females, mean age 62.4yr). After baseline cardiac assessment (CA), all received open-label G-CSF commencing at 10µg/kg s/c for 5 days, with an exercise stress test on days 4 and 6 (to facilitate myocardial cytokine generation and stem cell trafficking). After 3 months, CA and the same regimen of G-CSF+ESTs were repeated but, in addition, leukapheresis and a randomized double-blinded intracoronary infusion of CD133+ or unselected cells were performed. Final CA occurred 3 months thereafter. Eight events fulfilled pre-specified 'adverse event' criteria, but despite the poor outlook and co-morbidities of the patients, there were no deaths or adverse events resulting in long-term sequelae. Administration of consecutive cycles of G-CSF resulted in step-wise improvements in anginal frequency, EST performance and Duke treadmill scores (all $p<0.005$). Intracoronary infusion of CD133+ or unselected cells was safe but neither enhanced the response to G-CSF. Based on the results of GAIN-1, we have commenced a Phase II double-blind, crossover, placebo-controlled trial of G-CSF in 40 patients with refractory angina. While adverse events are not uncommon, G-CSF shows promise for the treatment of debilitating angina.

Peter MacDonald, MD, PhD, FRACP
Victor Chang Cardiac Research Institute, Sydney

Peter Macdonald is Conjoint Professor of Medicine in the University of New South Wales, Senior Staff Cardiologist, Cardiopulmonary Transplant Unit, St vincent's hospital and Co-head of the Transplantation Research Laboratory of the Victor chang Cardiac Research Institute. His major research interests over the last 10 years have been in the areas of treatment of end-stage heart disease, pulmonary hypertension, transplant allograft rejection and donor organ preservation injury. He has published over 120 original manuscripts in peer-reviewed scientific journals. He is a co-author of the Chronic Heart Failure guidelines for the National Heart Foundation/Cardiac Society of Australia and New Zealand. He is actively involved in both undergraduate and post-graduate medical education and has been a member of the National Examining Panel of the Royal Australasian College of Physicians. He chaired individual NHF RGICs in 2003, 2004 and 2005 and is currently national chair of the NHFs RGICs. He has contributed significantly to the conduct of the Chronic and Complex Care Programs in NSW. He has been a member of various Medicare Service and Pharmaceutical Industry-sponsored Advisory Committees and working parties involved in clinical trials and ongoing medical education of medical practitioners.

HUMAN NEURAL STEM CELLS: PRECLINICAL AND INITIAL CLINICAL EXPERIENCE

Human CD133+ CD34neg, CD45neg & CD24-/lo central nervous system stem cells expand exponentially in vitro when grown as neurospheres (HuCNS-SCns). They have been banked and tested in pre-clinical studies for their biological properties, safety and in models of efficacy. Upon transplantation into the brain of NOD-scid mice, HuCNS-SCns proliferate exclusively at host neurogenic sites, such as the sub-ventricular zone (SVZ) and the dentate gyrus of the hippocampus. Human cells resident in the SVZ maintain nestin expression, a marker for neural stem cells. Progeny of the human cells migrate to the olfactory bulb via the rostral migratory stream as chains of neuroblasts and continue to differentiate into neurons in the olfactory bulb. Cell migration also occurs extensively to cerebral cortex, basal ganglia, hippocampus, brainstem and cerebellum. The site-appropriate differentiation of HuCNS-SCns can be characterized by the combination of human specific monoclonal antibody, SC121 and lineage specific markers for neurons, astrocytes and oligodendrocytes. Engraftment has been durable and reproducible, with no evidence of tumour formation.

HuCNS-SCns can provide functional lysosomal enzyme that results in the protection of cortical and hippocampal neurons in a mouse model of Infantile Neuronal Ceroid Lipofuscinosis (NCL), a lysosomal storage disorder (LSD) caused by a deficiency of the enzyme, Palmitoyl Protein Thioesterase (PPT1). NCL or Batten Disease is a progressive fatal neurodegenerative disease affecting infants and young children. Studies of HuCNS-SCns transplanted into the brains of PPT1 KO/NOD-scid mice showed increased levels of PPT1 enzyme in the brain, decreased accumulation of pathologic autofluorescent lipofuscin material ($p < 0.0001$), and increased survival of host neurons ($p<0.001$) tested in the hippocampus. In addition, HuCNS-SCns transplantation extends the survival of PPT-/- mice compared to non-transplanted controls ($p<0.005$). SCI has initiated a Phase I trial to investigate the safety and preliminary efficacy of HuCNS-SC in Batten Disease. HuCNS-SC are transplanted directly into the brain. The trial is an open label study of two cell dose levels. The primary objective of the trial is to assess the safety of HuCNS-SC. The trial will also evaluate HuCNS-SC's ability to affect the progression of the disease. The primary evaluation will be at one year post HuCNS-SC transplant and patients continue monitoring for a five year period after transplantation. Since November 2006 three patients have received the low cell dose, and the first patient received the higher cell dose in July 2007. There have been no safety issues. Efficacy assessment for the first patient will occur in November 2007.

A second neuroprotection strategy is to reverse axonal damage due to oligodendrocyte deficiency or death. SCI have previously shown that HuCNS-SCns can remyelinate host axons when transplanted into the shiverer/NOD-scid mouse with a hypo-myelination defect. Confocal

analysis showed that the host axons were encircled with human myelin basic protein. At electron microscope level, these cells formed myelin sheath with 16-20 dense lines. Transplantation of HuCNS-SCns resulted in locomotor recovery in spinal cord injured immunodeficient mice, with evidence of remyelination provided by human cells.

Chris Juttner, FRACP

Consultant for StemCells Inc.

Chris Juttner is a clinical and regulatory consultant for StemCells Inc. He was involved in the initial development of Peripheral Blood Haemopoietic Stem Cell (HSC) transplantation while working at the IMVS, Royal Adelaide Hospital and Hanson Institute in Adelaide. He subsequently worked in HSC and Gene Therapy at SyStemix in Palo Alto, California from 1995 to 2000, and in Embryonic Stem Cell therapy at BresaGen from 2000 to 2004 and as a consultant for the Australian Stem Cell Centre from 2004.

PRECLINICAL AND CLINICAL APPLICATION OF MESENCHYMAL STEM CELLS

Adult (postnatal) mesenchymal stem cells (MSCs), derived from mammalian bone marrow, normally differentiate into bone, cartilage, muscle, adipose or stromal tissue. They also appear to be able to differentiate into tissues outside the mesenchymal lineage, including neuronal and hepatic tissue. They are therefore candidates for a broad range of therapeutic applications in regenerative medicine. MSCs are also non-specifically suppressive of T cell function and appear to decrease the incidence of graft-versus-host disease after allogeneic haematopoietic stem cell transplantation. Further exploration of their potential in organ transplantation and cell transplantation is merited.

Kerry Atkinson MD, FRCP, FRACP

Mater Hospital and Mater Medical Research Institute, Brisbane

Kerry Atkinson graduated in medicine from the Middlesex Hospital Medical School, University of London (UK). He underwent postgraduate training in oncology at the Royal Marsden Hospital in London. He was Assistant Professor of Medicine at the Fred Hutchinson Cancer Center /University of Washington in Seattle, USA working on the allogeneic bone marrow transplantation program. He joined the staff of St Vincent's Hospital, Sydney, Australia and spent 15 years helping develop the clinical and experimental bone marrow transplant program there. He served as President of the Transplantation Society of Australia and New Zealand, founded the Australasian BMT Cooperative Study Group and the Australian BMT Recipient Data Registry. From 1996-2003 he spent seven years in the USA cellular biotechnology industry, serving as Director of Clinical Transplantation at Systemix Inc, Palo and subsequently as Director of Cell and Gene Therapy for the American Red Cross. Prior to accepting the position of Director of Allogeneic Stem Cell Therapies at the Mater Hospital, Brisbane, he was Medical Director/Vice-President of Clinical Affairs at Osiris Therapeutics, Inc., Baltimore, USA and responsible for the company's clinical trial development program utilizing Universal Donor Mesenchymal Stem Cells in hematopoietic stem cell transplantation, repair of infarcted myocardium and meniscal regeneration. He has published 197 papers in the marrow transplant and stem cell literature, published "The BMT Data Book" (Cambridge University Press) and is the editor of the textbook "Clinical Bone Marrow and Blood Stem Cell Transplantation" also published by Cambridge University Press. He is a member of the NHMRC Cell Therapy Advisory Committee, a Member of the Advisory Committee of the Stem Cell Research Institute, National Health Research Institutes Taiwan , a Fellow of the Royal College of Medicine (UK), a Fellow of the Royal Australasian College of Medicine and a Professor of Medicine at the University of Queensland.

REGULATION OF THERAPEUTIC GOODS DERIVED FROM STEM CELLS: POLICY AND PROPOSED CLINICAL APPROACHES

The Therapeutic Goods Administration (TGA) has developed a regulatory framework for cell and tissue therapies which has been endorsed by the Australian Health Ministers Council. This presentation will describe the application of this framework to stem cells, and will also review ongoing proposals for clinical trials.

Albert Farrugia, PhD

Office of Devices, Blood and Tissues,
Therapeutic Goods Administration

Albert Farrugia is the Head of Blood and Tissue Services in the Office of Devices, Blood and Tissues of the Therapeutic Goods Administration (TGA), the Australian Commonwealth's regulatory agency for the pharmaceutical sector. He has been with the TGA since 1994, prior to which he had worked for 15 years in blood systems in Malta, the UK and Australia. Albert Farrugia received his BSc from the University of Malta in 1978 and his PhD from the Department of Transfusion Medicine in the University of Edinburgh in 1984. In 1988 he was elected a Fellow of the British Institute of Biomedical Sciences. He has had an active research career and has over ninety publications to his credit. He represents the TGA on the WHO Global Blood Safety Collaboration, the European Pharmacopeia Commission and the Council of Europe Committee Quality Assurance in Blood Transfusion. He is a member of the WHO's Expert Advisory Panel on Biological Standardisation. Besides his regulatory work he is a senior adviser on blood and tissues to the Australian Government's Department of Health and sits on various policy bodies. He is also a member of the Expert Advisory Panel of the World Federation of Haemophilia with responsibility for blood safety issues. He is also a Visiting Professor in the University of Western Australia and the University of Canberra. His primary interest is in developing appropriate regulatory and risk management strategies for the Australian blood and tissues sector. He is a keen philatelist and an enthusiastic but mediocre golfer, and has an interest in military history. Since 2006 he has assumed the role of an amateur artist, a pursuit which keeps him sane.

NSW Stem Cell Network

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