Newsletter

Australasian Gene Therapy Society Inc.

Message from the President

Dear AGTS members,

Due to a prior conference commitment, I was unable to attend the AGTS AGM on 29th April, held in The University of Technology, Sydney.

The end of 2009/beginning of 2010 was typically quiet with much time and effort devoted to the preparation and submission of NHMRC applications many of us have to repeatedly endure. One researcher, when asked about severe life disruptions that may have had an influence their career simply put down "RGMS". Enough said!

Since the last President's report, the committee has been settling in and planning future events to showcase Gene, Cell and Genetic Therapies in Australasia. Perhaps the most exciting development for 2010 will involve the 5th Australian Health & Medical Research Congress, to be held in Melbourne this November. The AGTS will be organising one session of gene and genetic therapies and another session, in conjunction with the HGSA, show-casing international (Profs Patrick Aubourg and Jay Neitz) and national speakers.

Planning the next AGTS conference is already underway, with Melbourne selected to host the meeting to be held 4-6 May, 2011. The final choice of venue is yet to be determined.

AGTS members have been in the media recently, with Prof John Rasko discussing stem cell therapies on ABC's catalyst in April, and later that month, there was a story on the dystrophin exon skipping therapy developed in Perth and being trialled in the UK to treat Duchenne muscular dystrophy.

Congratulations to the winners of the "Best Gene Therapy paper" by Allison Dane (Sexually Dimorphic Patterns of Episomal rAAV Genome Persistence in the Adult Mouse Liver and Correlation With Hepatocellular Proliferation published in *Molecular Therapy*) and Aparajita Khatri (Cytosine Deaminase-Uracil Phosphoribosyltransferase and Interleukin (IL)-12 and IL-18: A Multimodal Anticancer Interface Marked by Specific Modulation in Serum Cytokines published in *Clinical Cancer Research*).

The AGTS is a small society and we continue to explore links and associations with other like-minded societies, to promote and publicise Australasian

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gene and genetic therapy on the national and international stage. Similarly, we have been active in disseminating information on up-coming International and National meetings relevant to our members.

Finally, my sincere thanks to the team that make up the executive committee. Their tireless efforts in face of ever-increasing demands on their time will ensure we remain a strong and vibrant society.

Steve Wilton
President

"Best paper" Prize by an AGTS Member

Congratulations to AGTS members Drs Sharon Cunningham, Grant Logan (pictured) and Allison Dane all from the Gene Therapy Research Unit of the Children's Medical Research Institute in Sydney and Dr Aparajita Khatri from the Oncology Research Centre, Prince of Wales Hospital Clinical School, for their papers:

Cunningham *et al.* 2009, *Molecular Therapy* **17:** 1340-1346

Dane *et al.* 2009, *Molecular Therapy* **17:** 1548-1554

Khatri *et al.* 2009, *Cancer Clinical Research* **15:** 2323-2334

Logan *et al.* 2009, *Gene Therapy* **16:** 200–210

Don't Forget to Send us Your Papers!

To encourage our early career scientists and disseminate information among members, the AGTS Executive is offering two $250 awards twice a year. These will be offered in each 6 month period of a calendar year to the first author of a gene therapy publication judged by the Executive as the best for the previous period.

Only current AGTS members may apply and the award is open to all AGTS members (the young as well as the wise). Please send your paper details to our Secretary at sginn@cmri.org.au for consideration.

Gene Therapy Fulfilling its Promise

It has now been over 20 years since the first marker gene study was performed at the National Institutes of Health, which investigated both the feasibility and safety of gene transfer to human cells. Since then, clinical trials for the primary immunodeficiencies ADA-SCID and SCID-X1 are among the few disease targets where there has been clear evidence of clinical benefit. Below we highlight some of the more recent success stories, where treatment by gene therapy is translating into real therapeutic gains.

Stem cell gene therapy for X-linked adrenoleukodystrophy

Lentiviral vector-mediated gene transfer has been used successfully to halt the progression of adrenoleukodystrophy (ALD), a fatal neurodegenerative disease in two seven-year-old boys. Featured in the movie "Lorenzo's Oil," ALD is a severe hereditary condition caused by a deficiency of a protein involved in fatty acid degradation. Sufferers steadily lose their myelin sheath, the protective layer that coats nerve fibres in the brain. This disease is fatal without therapeutic intervention. Two years following treatment, gene-corrected cells were still detectable in both patients. Encouragingly, both patients showed neurological improvement and a delay in disease progression comparable to that seen with bone marrow transplants. Full details can been found in their *Science* paper:

Cartier *et al.* (2009). Haematopoietic stem cell gene therapy with a lentiviral vector in X-linked adrenoleukodystrophy. *Science* **326:** 818-823

AAV-mediated gene therapy for Leber congenital amaurosis

Researchers in a clinical trial lead by Professor Artur Cideciyan and conducted at the University of Pennsylvania and the University of Florida have treated three young adults by direct retinal injection of a recombinant AAV vector. These patients, who have been clinically blind since birth, suffer from a form of Leber congenital amaurosis resulting from mutations in the *RPE65* gene. One year following treatment, the patients have maintained their initial visual gains and an immune response to the vector has not been observed. These results provide evidence that the introduced *RPE65* gene is functional and can increase the light sensitivity of the retina. Full details can be found in their *New England of Journal of Medicine* paper:

Cideciyan *et al.* (2009). Vision 1 year after gene therapy for Leber's congenital amaurosis. *New England of Journal of Medicine.* **361:** 725-727

Gene therapy cures colour blind monkeys

Researchers, lead by Professor Jay Neitz from the University of Washington Medical School, have examined the feasibility of curing colour blindness in adult monkeys with a recombinant AAV vector encoding L-opsin. Red–green colour blindness, which results from the absence of visual photo-pigments, is the most common single locus genetic disorder. Twenty weeks post-treatment, the monkeys' colour skills improved and they were able to see in three colours or shades. The treated animals retained this ability for more than two years with no apparent adverse effects. The addition of the L-opsin protein was sufficient to restore colour vision despite the fact that the animals had been colour blind since birth. This study "provides a positive outlook for the potential of gene therapy to cure adult vision disorders." Full details can be found in their *Nature* paper:

Katherine Mancuso *et al. (2009). Gene therapy for red–green colour blindness in adult* primates. *Nature* **461:** 784-787

Lentiviral gene therapy for severe human β-thalassemia

In a clinical trial, lead by Associate Professor Philippe Leboulch (Brigham and Women's Hospital, Harvard Medical School) and carried out in Paris, an 18-year old male, lacking a HLA-matched sibling donor, was transplanted with autologous $CD34^+$ cells transduced *ex vivo* with a lentiviral vector expressing a marked b-globin transgene. The patient has been factor dependent since the age of three and prior to treatment required monthly transfusions. It has now been over two years since transplantation and greater than one third of their haemoglobin levels are vector-derived. No transfusion has been provided since twelve months post-treatment. Of interest, approximately half of the therapeutic effect is mediated by a dominant clone containing an integration within the *HMGA2* gene. Haematopoietic homeostasis is currently maintained, however, long-term follow up of this patient will establish whether the presence o this dominant clone results in any adverse clinical effects. Details of this study have been submitted for publication.

Would you like to Present at the AHMRC?

In conjunction with the HGSA, the AGTS has secured both Professor Jay Neitz and Professor Patrick Aubourg as keynote speakers at the upcoming 5th Australian Health and Medical Research Congress. These investigators head groups that have recently published papers in Science and Nature on the use of gene therapy for the treatment of colour blindness and X-linked adrenoleukodystrophy, respectively. In addition, the AGTS Executive is seeking six students or early-career post-docs (within three years of completion) to present their work in a gene therapy showcase session with an accompanying $250 award to off-set their expenses. Applicants must be current AGTS financial members. Please submit your abstracts, of no longer than 300 words, to the Secretary at sginn@cmri.org.au by Friday 16th July for consideration.

Dates for your Diaries

The XVIIIth Annual Congress of the European Society of Gene and Cell Therapy (ESGCT) in 2010 will be held from the 22nd to 25th October in Milan, Italy. Congress registration is now open and details are available on the ESGCT website at www.esgct.eu

The Australian Health & Medical Research Congress

The 5th AH&MR Congress will be held on the 14-18th November 2010 at the Melbourne Convention and Exhibition Centre. The AGTS is hosting a symposium on Wednesday 17th November 2010. Details are available on the congress website: www.ahmrcongress.org.au

Planning is now underway for the 7th biennial AGTS conference. Next year, the conference will be held in Melbourne from the 4th to the 6th May. More details to follow...

Australasian Gene Therapy Society Inc.

Good News for Australian Gene Therapy

Antisense oligomer-induced splice switching has been used to by-pass protein-truncating mutations in the huge dystrophin gene, that would otherwise lead to the severe muscle wasting disease, Duchenne Muscular Dystrophy. Two recent publications have shown how far this work has progressed since our first *in vitro* studies a decade ago.

Our colleagues in Oxford reported rescue of a severely affected mouse model of muscular dystrophy (Goyenvalle *et al.,* Molecular Therapy *advance online publication 20 October 2009).* The *dko* (utrophin and dystrophin deficient) mouse rarely lives beyond 12 weeks of age, whereas *mdx* (dystrophin deficient) mice only show obvious symptoms later in life (after 1 year). *Dko* mice treated with a morpholino coupled to a cell penetrating peptide to enhance uptake, designed to remove the mutated exon 23, showed a dramatic improvement, with near normal mobility and activity. This is the first time long-term benefits have been shown using antisense oligomer-induced splice switching in a severely affected mouse model of muscular dystrophy.

But enough of "mouse doctoring", the results of the first proof of concept trial, using a morpholino oligomer developed in Perth to by-pass the most common type of dystrophin mutation, have been published in Lancet Neurology (Kinali *et al.*, 2009, *Local restoration of dystrophin expression with the morpholino oligomer AVI-4658 in Duchenne muscular dystrophy: a single-blind, placebo-controlled, dose-escalation, proof-of-concept study* 8: 918-928). Restoration of dystrophin expression was observed in the injected muscle in a dose-dependant manner, with all participants exhibiting unequivocal targeted dystrophin exon 51 skipping that compensated for the disease-causing mutation. These results were sufficiently encouraging to gain approval to commence systemic administration of the morpholino at the beginning of the year. The dose-escalating study should be completed by the end of 2009, and results are eagerly anticipated.

The 6th AGTS Conference Delegates

The time since our biennial meeting held at the Kerry Packer Education Centre in Sydney on 29th April to the 1st May 2009 seems to have flown by. With the help from our sponsors, we were able to invite four international speakers and award many prizes to our attendees. We also awarded three life membership awards in recognition of outstanding work in the field of gene therapy. Professor Pamela Russell presented an excellent Greg Johnson Memorial Oration. In addition, we conducted a successful election to install a new Executive Council.

The Australasian Gene Therapy Society Members at the 6th Biannual Meeting 29th April - 1st May 2009 Kerry Packer Education Centre, Sydney, Australia.

Greg Johnson Memorial Oration by Professor Pamela Russell

Oncology Research Centre, Prince of Wales Hospital Clinical School, The University of New South Wales

A Perspective on Cancer Gene Therapy: Thirty years after its initiation, human gene therapy is now in an exciting new area of medical research, due to clearer principles of biology, progress in genomics and proteomics and developments in genetic engineering. Strategies for cancer gene therapy are based on knowledge of the biology of the particular cancer, and include targeting a defective gene, such a tumour suppressor gene, upregulation of drug resistance genes on normal tissues in conjunction with chemotherapy, down-regulation of specific genes through the use of siRNA, or targeted killing within a non-essential organ, such as the prostate through gene-directed enzyme drug therapy Alternatively, strategies are developed to upregulate anti-cancer immune responses. With respect to cancer, development of delivery vehicles that are efficient at allowing the gene to reach target tissues at adequate levels and specific to only target cancer tissues with appropriate regulation to protect the normal tissues, have formed the main thrust of current research. Thus, improving the potency and specificity of therapeutic delivery and increasing safety profiles remain major challenges in clinical oncology. Cellular therapies provide the perfect arena to allow systemic and targeted delivery of gene vectors or even better, nanoparticles together with gene vectors. New directions that will lead to the final culmination of gene therapy as stand alone or a potent adjuvant therapy to current therapies were highlighted.

Professor Pamela Russell (centre) with committee members Drs Gerald Both and Rosetta Martiniello-Wilks

"improving the potency and specificity of therapeutic delivery and increasing safety profiles remain major challenges in clinical oncology."

Life Membership

Congratulations to Dr Gerry Both and Professors Ian Alexander and Pamela Russell, who all received life-time membership to the AGTS at the 6th AGTS conference in Sydney last year for recognition of their outstanding efforts and achievements in gene therapy.

Marguerite Evans-Galea Recipient of the Panos Ioannou Young Investigator Award

On April 14th, 2005 the Executive Members of the AGTS on behalf of its Members established the Panos Ioannou Young Investigator Award in recognition, appreciation and thanks to Panos for his founding, passionate and sustained support for young scientists in the society. At the closing session of the 6th AGTS meeting, Dr Marguerite Evans-Galea received the Panos Ioannou Young Investigator Award which was this year was sponsored by the Journal of Gene Medicine.

Recipient of the Young Investigator Award Dr Marguerite Evans-Galea with Dr Rosetta Martiniello-Wilks and Professor Steve Wilton

I extend my sincere thanks to all members of the AGTS Executive Committee (and the society as a whole) for welcoming me into the membership so warmly. To receive the Panos Ioannou Young Investigator Award is a great honour and privilege that was most unexpected.

Focussing on the lipid oxidative stress response in lower eukaryotes, I obtained my PhD from the University of New South Wales. From 1999 to 2007, I gained extensive postdoctoral experience in the USA. At the University of Utah I characterised proteins involved in iron and zinc homeostasis. In 2001 I accepted a fellowship at St. Jude Children's Research Hospital in Memphis, Tennessee where I demonstrated improved lentiviral vector safety upon incorporation of an insulator. In 2008, I returned to my native Australia to join the Bruce Lefroy Centre for Genetic Health Research at the Murdoch Childrens Research Institute (MCRI). I have initiated two novel projects focused on identifying unique regulatory factors and developing potential diagnostic markers and therapies for the neurological disorder Friedreich ataxia.

My passion for gene therapy of Friedreich ataxia is akin to Prof. Ioannou's own work and I have heard many anecdotal stories of his time here at the Murdoch Childrens Research Institute. Several of his fellows are now staunch colleagues and I have very much enjoyed our collaborations! It is my hope to live-up to Prof. Ioannou's high standards and that of the Australasian Gene Therapy Society. To have the support of this close, cohesive group is invaluable and something I will treasure professionally and personally.

Marguerite Evans-Galea, Murdoch Childrens Research Institute

Prizes for Best Abstracts by Students

The AGTS has awarded student prizes at each biennial conference since the inaugural meeting. The poster sessions have grown over the years with the recent meeting exhibiting 27 posters in a new poster "walk-about" format. At the closing session five prizes were awarded to student members in recognition of their excellence in research.

(P5) RESTRICTION OF RD114 PSEUDOTYPED VECTORS IN BABOON CD34+ HSC

Jennifer M. Randall,[1] Stephen R. Larsen,[1,2,3] Rosetta Martiniello-Wilks[1,3] and John E.J. Rasko[1,3], [1]*Gene and Stem Cell Therapy Program, Centenary Institute and University of Sydney,* [2]*Institute of Haematology, Royal Prince Alfred Hospital (RPAH),* [3]*Cell & Molecular Therapies, Sydney Cancer Centre, RPAH, NSW, Australia*

(P9) TARGETING OF PLASMID DNA-LIPOPLEXES TO CELLS WITH MOLECULES ANCHORED VIA A METAL CHELATOR LIPID

Thomas P. Herringson, Ram R. Patlolla and Joseph G. Altin, *Biochemistry and Molecular Biology, School of Biology, College of Medicine, Biology and Environment, The Australian National University, Canberra, ACT, 0200, Australia*

(P13) LENTIVIRAL-MEDIATED GENE TRANSFER OF ANTICD4 SCFV PROLONGS CORNEAL ALLOGRAFT SURVIVAL

Sarah L. Brice, Lauren A. Mortimer, Claire F. Jessup, Kirsty A. Marshall, Helen M. Brereton and Keryn A. Williams, *Department of Ophthalmology, Flinders University, Adelaide Australia*

(P15) RISK ACCEPTABILITY IN CLINICAL RESEARCH: IS GENE THERAPY TOO RISK-AVERSE?

Claire T. Deakin,[1,2] Ian Kerridge[2,3] and Ian E. Alexander[1,4], [1]*Gene Therapy Research Unit, Children's Medical Research Institute and the Children's Hospital at Westmead.* [2]*Centre for Values, Ethics and the Law in Medicine, Faculty of Medicine, University of Sydney.* [3]*Department of Haematology, Westmead Hospital.* [4]*Discipline of Paediatrics and Child Health, University of Sydney*

(P25) OPTIMISATION OF ANTISENSE OLIGONUCLEOTIDE COCKTAILS USING *IN SILICO* AND *IN VITRO* TECHNIQUES FOR TARGETED EXON SKIPPING IN THE DYSTROPHIN CENTRAL ROD DOMAIN

Lucy Barrett,[1] Gavin Pinniger,[2] Abbie M. Fall,[1] Sue Fletcher[1] and Steve D. Wilton[1], [1]*Molecular Genetic Therapy Group, Centre for Neuromuscular and Neurological Disorders, University of Western Australia.* [2]*School of Biomedical, Biomolecular and Chemical Sciences, University of Western Australia*

Life members Professor Ian Alexander, Dr Gerald Both and Professor Pamela Russell (back row) and prize winners Thomas Herringson, Claire Deakin, Lucy Barrett, Jennifer Randall, Dr Marguerite Evans-Galea and Sarah Brice

International Speakers at the AGTS meeting

The AGTS is committed, with the assistance of sponsors, to inviting speakers who are international leaders in Gene Therapy to our biennial meetings. Four such speakers were featured this year. Summaries of their presentations have been included below. The opportunity to meet, discuss research and collaborations with these speakers was particularly valuable for early career scientists and younger members of our society.

Professor Anthony Gringeri

Amsterdam Molecular Therapeutics,
Amsterdam, The Netherlands.

Title: Adeno-associated viral vectors in clinical trials of lipoprotein lipase deficiency

Over the last twenty years, gene therapy has become a practical clinical tool partly because of advances in vector technology. Adeno-associated viral vectors (AAV) are nonpathogenic, replication-deficient parvoviruses that transfer DNA most efficiently to tissues with a low turnover rate, e.g., muscle, brain, liver, and retina. The transgenes then remain mainly episomal. Individual AAV serotypes display distinct tissue tropisms, allowing the transduction of target tissues with great specificity. Therapeutic effect with a variety of transgenes has persisted lifelong in laboratory animals. AMT has performed two clinical trials using the transgene encoding lipoprotein lipase (LPL), the enzyme catalyzing triglyceride (TG) catabolism. Patients with LPL deficiency have abnormally high serum TG concentrations and chylomicronemia, which may cause life-threatening pancreatitis (PT). In these clinical trials, the majority of patients showed a reduction in serum TG as well as improvement in other symptoms like eruptive xanthomas and lipemia retinalis. Analysis of muscle biopsies showed that expression of active LPL protein correlated well with the number of genome copies administered. Furthermore, tissue staining revealed LPL-mediated lipid uptake in injected muscle, whereas no uptake was seen in non-injected muscle from the same patients. Most importantly, treated patients showed a nearly seven-fold reduction in the incidence of pancreatitis. Treatment was well tolerated, with an acceptable safety profile. Thus, AAV are a safe and effective means to mediate gene therapy in humans.

Dr. Carolyn Breitbach

Jennerex Biotherapeutics Inc., San Francisco, CA and Department of Clinical Pharmacology, University of Oxford

Title: Targeted and armed oncolytic Poxviruses: a novel multi-mechanistic therapeutic class for cancer

Engineered viruses have been developed for cancer therapy both as non-replicating gene therapy agents and as cancer vaccines. Oncolytic viruses, in contrast, were developed to replicate within, and subsequently lyse, cancer cells. Clinical efficacy to date with each of these approaches has been limited by multiple factors including the inability to infect enough tumor cells *in vivo* locally within a tumor or systemically, and resistance of complex advanced tumors to a single mechanism-of action (MOA). Over the last several years, however, a novel therapeutic class has emerged that combines the best features of all three approaches: targeted and armed oncolytic poxviruses. Recent preclinical and clinical results demonstrate convincingly that products from this therapeutic class can achieve highly selective and potent cancer destruction systemically through a multipronged MOA. Given recent clinical validation, we expect this therapeutic class to expand rapidly.

Professor Jeffery Chamberlain

Department *of Neurology, Muscular Dystrophy Research Center, University of Washington Medical School, Seattle*

Title: Development of gene therapy for Duchenne muscular dystrophy using systemic delivery on AAV vectors expressing truncated dystrophins

Duchenne muscular dystrophy (DMD), is caused by mutations in the dystrophin gene. We are developing methods to deliver therapeutic genes to muscles throughout the body to either replace the missing dystrophin gene or to help compensate for the lack of dystrophin. We show that shuttle vectors derived from adeno-associated virus type 6 (rAAV6) are able to deliver genes to muscles throughout the body of adult mice when injected directly into the bloodstream. rAAV6 delivery results in highly efficient gene expression in skeletal and cardiac muscle that persists for the lifespan of the mouse. However, the AAV shuttles have a limited carrying capacity, and as a result we have also been developing truncated versions of the dystrophin gene that can be carried by AAV yet retain sufficient functional capacity to halt dystrophy. Subtle modifications to the design of microdystrophin result in dramatic differences in properties such as force development, neuromuscular junction maturation, myotendinous junction fragility and formation of ringed myofibers. A single injection of an AAV6/micro-dystrophin vector into the vasculature of adult, dystrophic mice results in elimination of dystrophic histopathology for the lifespan of the mouse. We are also focused on scaling up the procedures in the dog model of DMD. These studies revealed a cellular immune response directed against the AAV capsid proteins, but which could be blocked by short-term immune suppression, leading to long-term dystrophin expression. We have therefore begun testing whether AAV6 vectors can be delivered via the vasculature to dogs. We have observed that delivery of AAV6 vectors into various veins and arteries of the dog results in efficient gene transfer to downstream muscles, but does not lead to whole body gene transfer. Instead, it appears that vector will need to be delivered into multiple vascular sites to target muscles body wide. These results suggest that a combination of intravascular AAV delivery coupled with transient immune suppression could lead to an effective therapy for DMD.

Dr. Shahriar Yaghoubi

Molecular Imaging Program, Department of Radiology, School of Medicine, Stanford University, USA

Title: Application of molecular imaging

Molecular imaging is enabling non-invasive analysis of therapeutic transgene (TG) or therapeutic cell (TC) pharmacokinetics through time in living subjects, including humans. The expression of a TG can be imaged either directly using a specific imaging probe that detects the products it encodes, or indirectly by linking its expression to that of an imaging reporter gene (RG). Indirect TG expression can be accomplished by co-vector administration or designing a fusion TG-RG genetic construct, a bicistronic construct containing the TG and RG under control of the same promoter, but separated by an internal ribosomal entry site, a single genetic construct containing both RG and TG with double identical promoters, and a bidirectional transcriptional approach. All of these techniques have been studied successfully in pre-clinical models. The direct approach has been used to monitor suicide gene therapy with Herpes Simplex Virus 1 thymidine kinase (HSV1-tk, or its mutants) in rodents with implanted tumors and in patients with hepatocellular carcinoma. Three different techniques have so far been used, evaluated or envisioned for imaging TCs in living subjects. Direct labeling of cells with imaging probes is the simplest technique and has been demonstrated in humans using radionuclide or MRI based probes. Another technique that involves genetically engineering TCs with a RG and then imaging them with a reporter probe (RP) can allow long-term imaging of all aspects of TC pharmacokinetics (biodistribution, survival, status) at multiple time points in living subjects. We recently reported the first case of imaging cytolytic T cells homing to glioblastomas, using the HSV1-tk RG and the positron emission tomography RP, [18F]FHBG in a patient (Nature Clinical Practice Oncology 6(1):53–58 (January 2009)). Finally, one can envision developing a new probe that can specifically detect a receptor associated with the therapeutic cells. Molecular imaging should help optimize cell/gene therapy protocols for patients.

ESGCT Meeting Report

The XVIIth Annual Congress of the European Society of Gene and Cell Therapy (ESGCT) was held this year in Hannover, Germany, in conjunction with the 4th Annual Congress of the German Society for Stem Cell Research (GSZ) and the 16th Annual Meeting of the German Society of Gene Therapy (DGGT). Immediately preceding the congress, also held in Hannover, was the 5th Stem Cell Clonality and Genotoxicity Retreat (SCCGR). The conference this year covered the entire spectrum of somatic gene therapy, stem cell biology and regenerative medicine with almost 90 invited presentations, a similar number of oral presentations from selected abstracts and over 350 posters.

There were some exciting updates on recent clinical progress including the conversion of one patient with severe human beta-thalassemia to transfusion independence presented by Philippe Leboulch and the results of three patients receiving haematopoietic stem cell therapy for X-linked adrenoleukodystrophy by Nathalie Cartier, both using lentiviral vector gene delivery. This latter study has also been recently published in the 6th November issue of *Science* (Haematopoietic stem cell therapy with a lentiviral vector in X-linked adrenoleukodystrophy, 2009, Science 326 (5954): 818-823).

Progress in the field of induced pluripotent stem cells (iPS) was on display with a number of sessions highlighting the enormous potential of the use of these cells. Presentations included the induction of pluripotency in adult stem cells by Hans Robert Schöler (Germany), optimised culture conditions for reprogramming mouse fibroblasts by defined factors presented by Duanquing Pei (China) and advances of gene therapy and cell reprogramming in Fanconi anaemia by Juan Bureren (Spain).

The Presidential Symposium this year was a highlight with a presentation by David Klatzmann (Paris, France) accounting his experience in he field of HIV and AIDS research entitled *"The early days of AIDS/HIV research: Extraordinary times, (extra)ordinary people"*. Our congratulations also go out to Professor Marina Cavazzana-Calvo who received the inaugural *Outstanding Achievements Award* for her work in the field of gene therapy targeting diseases of the haematopoietic system, including X-linked severe combined immunodeficiency first reported almost ten years ago now in *Science*. With such advances in the fields of gene and cell therapy, success in the clinic now seems closer than ever. Abstracts from the meeting are published in the November issue of *Human Gene Therapy* (20 (11): 1351-1558).

AGTS Executive Committee

President:	Professor Steve Wilton
Vice-president:	Dr Rosetta Martiniello-Wilks
Treasurer:	Professor Ann Simpson
Secretary:	Dr Samantha Ginn
Executive members:	Dr Paul Gregorevic
	Professor Elizabeth Rakoczy
	Dr Jim Vadolas
Ex-officio member:	Dr Gerry Both

The Australian Health & Medical Research Congress

14-18th November 2010
Melbourne Convention and Exhibition Centre

Australasian Gene Therapy Society (AGTS)

Wednesday 17th November 2010

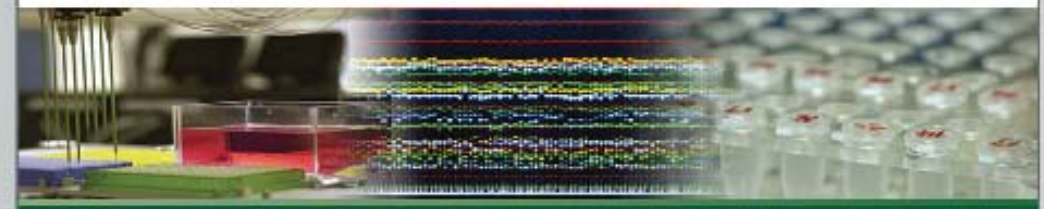
Sessions

New genetic therapies & therapeutic technologies (Part A) (jointly with HGSA)
New genetic therapies & therapeutic technologies (Part B)

Invited Speakers

Dr Patrick Aubourg, University of René Descartes, FR
Prof Jay Neitz, University of Washington Medical School, USA
A/Prof Frank Alderuccio, Monash University
Prof Ian Alexander, University of Sydney
Prof Alan Mackay-Sim, National Centre for Adult Stem Cell Research
Prof Pamela Russell, Queensland University of Technology
Dr Paul Verma, Monash Institute of Medical Research
Prof Steve Wilton, University of Western Australia

For more information visit the website below



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