## Australasian Gene Therapy Society Inc.

#### Message from the President

One of our Society highlights for 2013 included the 8th AGTS conference held here at the University of Technology, Sydney, Aerial Function Centre, 8 –10<sup>th</sup> May. With the support of our sponsors Aspen Australia, Children's Medical Research Institute, the Australian Neuromuscular Research Institute, ThermoFisher Scientific, Genzyme, Bioline, Life Technologies, Sigma-Aldrich, VWR, Lonza, and the Journal of Gene Medicine-Wiley/Blackwell we were able to invite four world renown International speakers, as well as many high profile Australian researchers.

Several gene therapy trials were showcased, now making substantial differences in "clinical trial" patients with blood and muscle disorders, compromised immune systems and eye diseases. We



heard about the exciting Phase I/II haemophilia B clinical trials by Prof Edward Tuddenham and Dr Amit Nathwani from University College Hospital London (UCL). These studies utilised adeno-associated virus (AAV) 8 to deliver the Factor IX gene directly into the patient's liver to cure patient bleeding disorders. Prof Nathalie Cartier from INSERM France, described her innovative gene therapy strategies for genetic leukodystrophies. Australian researchers Prof Geoff Symonds of Calimmune P/L described his Phase II clinical trial for cell-delivered gene therapy for HIV and Dr Chooi-May Lai from the University of Western Australia described her group's results from a Phase I/II gene therapy clinical trial in patients with age-related macular degeneration. Our own Professors Steve Wilton and Sue Fletcher described their Phase II trials in antisense oligomer induced splice switching in patients with muscular dystrophy, increasing induced protein and restoring the ability of adolescent boys to walk. The significance of this cutting edge work was recognised with the award of the Australian Museum Eureka Prize to both Steve and Sue in October 2013. Congratulations Steve and Sue.

# The success of 8th AGTS conference also lies with the tireless efforts of the AGTS Executive Committee for which I am sincerely thankful. I also thank Margot Latham, Samantha Gall and Dr Eileen McGowan for their assistance with the conference organisation. At the last 2013 AGTS AGM, the Executive Committee underwent a change to welcome Dr Marguerite Evans-Galea as a Committee Member with Prof Steve Wilton stepping down as President, now maintaining an ex-officio role. This my opportunity on behalf of the Executive to thank our past President for his leadership of our society, boundless energy and for being an inspiring role model showing hard work, perseverance and good humour are invaluable qualities for translating science successfully into the clinic. Many thanks also go to Dr Gerry Both who retired as an ex-officio member. The Executive Committee now consists of Dr Jim Vadolas (Vice President), Prof Ann Simpson (Treasurer), Dr Samantha Ginn (Secretary) and Dr Sharon Cunningham and Dr Paul Gregorevic as Executive members. Special thanks and congratulations go to Samantha for being an outstanding soci-

Finally, the AGTS is committed to promoting the growth and development of gene and genetic therapies to treat inherited and acquired disorders. We also strive to build stronger links with the cell therapy research community and endevour to reflect this initiative in our recent decision for a name change to the Australasian Gene and Cell Therapy Society (AGCTS) that we will discuss at this AGM. In addition, we strive to disseminate

ety Secretary and we welcome her new baby Noah to the AGTS family.

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information, share resources and expertise through updates and communications to our members and we also achieve this through advertising special seminars, events and functions through the Executive committee. We also seek to promote and encourage our researchers, and the AGTS Best Paper Prize seeks to acknowledge and reward the quality of research published by both our young and senior members. The winners in award rounds 1 and 2 in 2013 were:

Dr Binhai Ren from the Simpson Group, School of Medical & Molecular Biosciences University of Technology Sydney for "Long-term reversal of diabetes in non-obese diabetic mice by liver-directed gene therapy" published in the Journal of Gene Medicine.

Georg von Jonquieres from the Translational Neuroscience Facility, University of New South Wales for "Glial Promoter Selectivity following AAV-Delivery to the Immature Brain" published in PLOS ONE.

Dr Glen Reid from the Asbestos Disease Research Institute, Concord Hospital for "Restoring expression of miR-16: a novel approach to therapy for malignant pleural mesothelioma" published in Annals of Oncology.

Dr Geoff Symonds from Calimmune P/L Darlinghurst for "Pre-clinical Safety and Efficacy of an Anti-HIV-1 Lentiviral Vector Containing a Short 4 Hairpin RNA to CCR5 and the C46 Fusion Inhibitor" published in Molecular Therapy—Methods & Clinical Development.

If you are a current member, please submit your latest papers to Sam for consideration by the AGTS Executive.

The new AGTS Executive Committee have begun planning for the 9<sup>th</sup> AGTS Conference April 29 to 1 May 2015 in Melbourne. Please save this important date. This is a must-do event for medical researchers and clinicians fascinated by the recent progress in gene therapy and stem cell biology. We are expecting over 200+ professionals in gene therapy, cell-based therapies, RNA therapeutics, biomarkers and related fields to attend. On behalf of the AGTS Executive Committee, I look forward to welcoming you to the AGTS 2015 meeting in Melbourne next year.

With warm regards,

& Martmiello

Rosetta Martiniello-Wilks PhD

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President, Australasian Gene Therapy Society <a href="www.agts.org.au">www.agts.org.au</a>

### "Best paper" Prize by an AGTS Member

Congratulations to 2013 "best paper" prize winners Dr Glen Reid (University of Sydney), Dr Binhai Ren (University of Technology, Sydney), Professor Geoff Symonds (Calimmune) and Georg von Jonquieres (University of New South Wales). Please see above for full details of their manuscripts.

### Don't Forget to Send us Your Papers!

To encourage our early and not so 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 or corresponding author of a gene therapy or RNA therapeutic publication. 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 <a href="mailto:sginn@cmri.org.au">sginn@cmri.org.au</a> for consideration.

### Congratulations to AGTS members Professor Sue Fletcher and Professor Steve Wilton



Professors Steve Wilton and Sue Fletcher from Western Australia's Murdoch University were awarded the prestigious 2013 NSW Health Jamie Callachor Eureka Prize for Research Translation. Full details can be found at <a href="http://australianmuseum.net.au/2013-eureka-medical">http://australianmuseum.net.au/2013-eureka-medical</a> or watch the YouTube video at <a href="http://www.youtube.com/watch?v=2WAQxC6WCEs& feature=share&list=PLEh1S0YpN664wb00k\_HMFX8FzikFZHsp2">http://www.youtube.com/watch?v=2WAQxC6WCEs& feature=share&list=PLEh1S0YpN664wb00k\_HMFX8FzikFZHsp2</a>

### The AGTS is soon to become the AGCTS!

At the recent Annual General Meeting, the membership unanimously voted on the proposed name change of the Society to the Australasian Gene and Cell Therapy Society. Members will be notified by the Secretary once the change has been finalised. Thank you to those members who attended the meeting.

### **Dates for your Diaries**

The XXII<sup>nd</sup> ESGCT and NVGCT Collaborative Congress will take place at The Hague, The Netherlands, from 23<sup>rd</sup> to 26<sup>th</sup> October 2014. Details on how to register are available on the ESGCT website at www.esgct.eu





The 7th AH&MR Congress will be held on the 16th-19th November 2014 at the Melbourne Convention & Exhibition Centre. Details are available on the congress website at www.ahmrcongress.org.au

The Australasian Stem Cell Science and Therapy Meeting held on the 9th-11th November 2014 at the Mantra, Lorne, Victoria. See www.asscr.asnevents.com.au for details











Planning is now underway for the 9th biennial AGTS conference. Next year, the conference will be held in Melbourne from the 29<sup>th</sup> April to the 1<sup>st</sup> May 2015. Please check the AGTS website for more details at www.agts.org.au

### The 8th Biennial AGTS Conference

The time since our biennial meeting, held at the Aerial Function Centre, University of Technology, Sydney (UTS) on 8th to 10th May 2013, seems to have flown by. With the help from our sponsors, we were able to invite an impressive lineup of International and National speakers; Professors Frederic Bushman, Nathalie Cartier, Bobby Gaspar, Edward Tuddenham, Martin Pera, Roger Reddel and Paul Reynolds. The meeting was opened with an acknowledgement to the Traditional Gadigal and Guring-gai people of the Eora Nation on which UTS stands. Proceedings were opened by Dr Fiona Cameron, Executive Director for Biological Sciences, Australian Research Council (ARC) pictured front left.

Again, with help from our sponsors, we were able to award prizes to our student members Lucy Barrett (WA), Timothy Colgan (VIC), Julia Suurbach (NSW) and Dr Virginia Wootton (NSW). Dr Cindy Kok (Gene Therapy Research Unit, Children's Medical Research Institute, Sydney) received the Panos Ioannou Young Investigator Award. Professor Roger Reddel presented an excellent Greg Johnson Memorial Oration, and we conducted a successful election to install a new Executive Council, with Steve Wilton stepping down as President and Marguerite Evans-Galea joining the Executive Committee. Gerry Both also stepped down from his ex-officio role on the Committee and we thank him for his time and continual support of the Society.



National and International delegates at the 8th Biennial Australasian Gene Therapy Society Meeting, 8th-10th May 2013, Aerial Function Centre, University of Technology Sydney, Australia.

### Meeting Sponsors, without whom this conference would not have been possible









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### UTS hosts the biennial Australasian Gene Therapy Society (AGTS) conference

The Australian Gene Therapy Society hold research conferences to discuss and explore new cutting edge technology and experimental techniques to treat, prevent and cure incurable diseases. The AGTS conference for 2013 was held 8-10 May at the University of Technology, Sydney (UTS).

Coordinated by the AGTS Executive Committee, the conference covered many topics from DNA integration in human gene therapy to genetic and cell therapy for a range of diseases and disorders. Keynote speakers and participants consisted of both international and local talent covering a broad spectrum of gene therapy research, technologies and applications.

One of the guest speakers was Dr Glen Banks from the University of Washington, USA. Originally from Australia, Dr Banks studied in Brisbane but has spent over ten years working and researching gene therapy for muscular dystrophy at the Department of Neurology at the University of Washington.

Dr Banks said "This is a great opportunity for all gene therapy researchers to come together to share knowledge and information about their research findings."

"Having this open forum allows other researchers in different gene therapy fields to ask questions and see if they can apply the same or similar techniques to develop or advance their own research."

"It's also great to promote awareness of gene research to the public for future funding and provide advice and information to clinicians who treat patients with gene diseases and disorders," Dr Banks said.

Professor Steven Wilton from Australian Neuro-Muscular Research Institute at Murdoch University WA also attended the conference. He was part of the conference organising committee and this is what he had to say about the event, "Sharing information and make collaborative connections is what it's all about."



"This type of conference gives you [researchers] a chance to sit down and think about work and let your mind absorb what other people are doing and how your work could impact on it, how you can improve on what you're doing [in your own research] as well as looking at the new lines of research."

"One of the key roles of these conferences to make collaborative connections because the type of work we're doing is applicable to many different conditions from diabetes, to stroke, to blood disease, to cancer and muscular dystrophy."

"It's about approaching the problem from different angles, finding out what other researchers are doing and adopting those findings or breakthroughs to your research. It's about opening the conversation up to give feedback or help other researchers improve their research in order to find a treatment, prevention or cure for different gene diseases," said Professor Wilton.

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### Greg Johnson Memorial Oration by Professor Roger Reddel

#### Genetic basis of telomere disorders

Roger Reddel, Christine Napier, Kylie Bower, Jane Noble, Lily Huschtscha, Rebecca Dagg, Loretta Lau, Michael Stutz and Hilda Pickett

Cancer Research Unit, Children's Medical Research Institute, Westmead, NSW 2145

The cell division cycle of normal human somatic cells is accompanied by telomere shortening, with sets an upper limit on cellular proliferative capacity. In highly proliferative tissues, such as the bone marrow, this is partially counteracted by a telomere lengthening mechanism (TLM), primarily the enzyme telomerase which reverse transcribes new telomeric sequence. There is a growing list of clinical disorders associated with excessive telomere shortening. Common features include bone marrow failure, disorders of skin and mucous membranes, and pulmonary fibrosis, but a wide variety of organs may be affected and in severe cases there may also be developmental defects. Known genetic causes of short telomere syndromes include mutations in any of the genes that encode subunits of the telomerase enzyme ribonucleoprotein complex, proteins involved in its biogenesis, a protein required for its trafficking to the telomere, a protein involved in processing telomere ends, or a component of the shelterin telomere-binding protein complex.



Cancer is also a telomeric disease: almost all cancers have an upregulated TLM which completely counteracts normal telomere shortening thus permitting unlimited proliferation. The upregulated TLM is telomerase in 85% of cancers and Alternative Lengthening of Telomeres (ALT) mechanism in most of the remainder. How these TLMs become upregulated is incompletely understood, but activation of telomerase is commonly associated with a mutation in the promoter for the gene encoding its reverse transcriptase subunit, TERT. Activation of ALT is often associated with mutations in the ATRX gene which encodes a protein involved in chromatin remodelling. This may present opportunities for therapeutic in-

Professor Roger Reddel, Director of the Children's Medical Research Institute, presenting the 2013 Greg Johnson Memorial Oration. We thank the Children's Medical Research Institute for its continual support of our meetings

### Dr Cindy Kok Recipient of the 2013 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 8th AGTS meeting, Dr Cindy Kok (Gene Therapy Research Unit, Children's Medical Research Institute, Sydney) received the Panos Ioannou Young Investigator Award to be used for conference travel expenses up to the value of \$2,500.

"It was a great honour and privilege to receive the Panos Ioannou Young Investigator Award. I would like to thank the AGTS Executive Committee for their support and also my supervisor Professor Ian Alexander for encouraging me to present my work at this year's conference. The prize money was used for travel costs to the Japan Society of Gene Therapy 19<sup>th</sup> Annual Meeting in Okayama, Japan.

From the talks which were presented at both the AGTS and JSGT meetings, I felt that this was an exciting time for the gene therapy field as there was a strong focus on translation of pre-clinical research and the recent outcomes in various clinical trials. I had always hoped to undertake a post doctoral position in Japan and this trip allowed me to share my work with the Japanese gene therapy community and also to

meet scientists and clinicians who also focus on liver-targeted gene transfer. This was a valuable and timely networking experience, as I found out during this time that I had also been awarded the JSPS Postdoctoral Fellowship for Foreign Researchers. I will therefore be undertaking a postdoctoral position at the University of Tokyo at the end of this year. I would like to thank the AGTS for helping to develop my research career and look forward to sharing my work again at future meetings."



Newly elected AGTS President Rosetta Martiniello-Wilks presenting the Panos Ioannou Young Investigator Award to Dr Cindy Kok

### **AGTS Student Prizes**



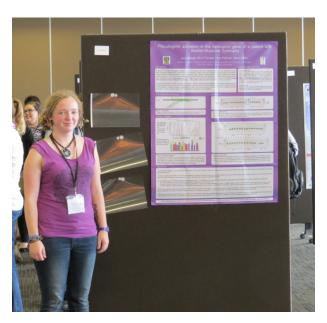
Timothy Colgan (Laboratory for Muscle Research and Therapeutic Development, Baker IDI Heart and Diabetes Institute, Melbourne) was awarded the \$250 Journal of Gene Medicine Student Oral Presentation Prize



Virginia Wootton (Gene Therapy Research Unit, Children's Medical Research Institute) was awarded a \$250 Student Poster Prize



Julia Suurbach (Translational Cancer Research Group, University of Technology, Sydney) was awarded the \$250 Journal of Gene Medicine Student Oral Presentation Prize



Lucy Barrett (Australian Neuromuscular Research Institute, The University of Western Australia, Perth) is awarded a \$250 Student Poster Prize

### 2013 AGTS Conference Student Award—Julia Suurbach

First of all I'd like to take this opportunity to thank the AGTS Executive and their Conference Sponsors Genzyme and the Journal of Gene Medicine-Wiley Blackwell Publishing for their generous support of the 2013 AGTS Conference Student Prizes. I received one of two Best Student Oral Presentation Awards (\$250) which

supported my travel to attend the Australian-Canadian Prostate Cancer Research Alliance Symposium at the Sheraton Mirage Resort, Port Douglas, Queensland in August 2013. This conference not only took place in paradise, but also gave me the opportunity to meet highly renowned experts in the field of prostate cancer research. It was especially lovely to meet YZ Wang from Vancouver Prostate Centre. We chatted about his exciting research identify-



ing new biomarkers to improve the diagnosis and prognosis of various cancers. I also had a good time discussing more

philosophical questions about science in general with YZ. It was also my pleasure to meet Professor Colleen Nelson, Professor Dietmar Hutmacher and my PhD supervisor's Dr Rose Martiniello-Wilks, former boss Professor Pamela Russell and her lab group. My personal highlight of this

meeting was the selection of my poster by the Conference Poster Judging Committee and an invitation for an oral presentation. This opportunity allowed me to explain my work to all conference attendees and I received helpful feedback and comments from the audience.

### Newly Elected AGTS Executive Committee



From left, Dr Marguerite Evans-Galea (Committee member), Dr Jim Vadolas (Vice President), Dr Gerry Both (out-going exofficio), Professor Steve Wilton (ex-officio), Dr Sharon Cunningham (Committee member), Dr Samantha Ginn (Secretary), Professor Ann Simpson (Treasurer), Dr Rosetta Martiniello-Wilks (President) and Dr Paul Gregorevic (Committee member)

### Conference Dinner Cruise on Sydney Harbour



From the left, AGTS life-member Dr Gerry Both, Amy Banks and Dr Glen Banks, Department of Neurology, University of Washington, Seattle



From the left, out-going AGTS President Professor Steve Wilton and invited speakers Professor Nathalie Cartier, INSERM France and Professor and Fredric Bushman, Perelman School of Medicine, University of Pennsylvania, Philadelphia



From the left, Nahal Haddadi, Rosaline Habib both from the University of Technology Sydney, and Mary Wong from Westmead Hospital



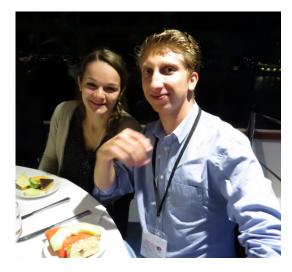
From the left, Richard Limburg, AGTS Treasurer Professor Ann Simpson from the University of Technology Sydney and Committee member Dr Sharon Cunningham, Children's Medical Research Institute, Westmead



From the top left, Mark Roosjen, Dr Bradley McColl, in-coming AGTS Vice-President Dr Jim Vadolas, Tiwaporn Nualkaew, Panjaree Siwaponanan and Betty Kao all from the Murdoch Childrens Medical Research Institute, Melbourne



From the left, AGTS student members Lucy Barrett from the Australian Neuromuscular Research Institute, Western Australia and Timothy Colgan from the Baker IDI Heart and Diabetes Institute, Melbourne



AGTS student members Julia Suurbach and Dario Gerace both from the University of Technology Sydney



From the left, Dr Susan Siew, Gustavo de Alencastro and Dr Virginia Wootton all from the Children's Medical Research Institute, Westmead