



Australasian Gene Therapy Society

7th Biennial Meeting 4 – 6 MAY

**Bio21 Institute
30 Flemington Road,
University of Melbourne, Parkville,
VIC, 3010, Australia.**

AGTS Organising Committee:

Prof Steve Wilton
Dr Rosetta Martiniello-Wilks
Prof Ann Simpson
Dr Samantha Ginn
Dr Jim Vadolas
Dr Paul Gregorevic
Dr Gerry Both
Dr N Tran
Dr E McGowan
Prof T Preiss
Prof N McMillan

7th AGTS Meeting SCIENTIFIC PROGRAM

Bio21 Institute, 30 Flemington Road, University of Melbourne,
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WEDNESDAY, 4th MAY, 2011

- 08.00-08.45 Registration, Poster set-up (before 9am)
Atrium Bio21 Institute
- 09.00-09.15 Welcome and Opening Address**
Bio21 Auditorium 1
AGTS President, Prof Steve Wilton (CNND, University of WA)
Deputy Director Bio21 Institute, Prof Malcolm McConville
- 09.15-11.00 Scientific Session 1: RNA-based Therapy (Benitec)**
Chair: Dr Peter French (CEO Benitec Limited)
- 09.15-10.00 **Keynote Speaker 1: Prof John Rossi**, *Small RNA therapeutics for the treatment of HIV* (Beckman Res. Inst., USA)
- 10.00-10.30 **Invited Speaker: Prof Maria Kavallaris**, *Targeting drug resistance in lung cancer* (CCIA, Sydney)
- 10.30-10.45 O1: Dr Leszek Lisowski, *Novel anti-HCV therapy: single shRNA targeting both strands of HCV* (Young Investigator, Stanford Uni. USA)
- 10.45-11.00 O2: Dr Carl Adkin, *Potential treatment of 'leaky' Duchenne Muscular Dystrophy mutations in the 5' region of the dystrophin gene using antisense oligomers* (Young Investigator, CNND, University of WA)
- 11.00-11.30 Morning Tea, Atrium Bio21 Institute**
Trade Display & Poster Viewing
- 11.30-13.00 Scientific Session 2: Cell & Gene Therapy**
Chair: Dr Gerry Both (Broadvector Ltd)
- 11.30-12.15 **Keynote Speaker 2: Prof Alan Mackay-Sim**, *Patient-derived stem cell models of brain diseases* (Eskitis Inst., Brisbane)
- 12.15-12.30 O3: Professor Ann Simpson, *Reversal of diabetes in mice following transplantation of an insulin-secreting liver cell line: Melligen cells* (UTS, Sydney)
- 12.30-12.45 O4: Dr Frank Alderuccio, *The utilization of gene therapy of haematopoietic stem cells as a strategy to treat autoimmunity* (Monash University, Melbourne)
- 12.45-13.00 O5: Dr Christopher Siatskas, *Migration and cellular localization of transplanted human mesenchymal stem cells in a mouse model of autoimmune demyelination* (Monash, Melbourne)
- 13.00-13.15 O6: Dr Rose Martiniello-Wilks, *Advances in cellular therapeutic gene transfer targeting prostate cancer metastases* (UTS, Sydney)

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WEDNESDAY, 4th MAY, 2011 (continued)

- 13.15-14.00** Lunch, Atrium Bio21 Institute
Trade Display & Poster Viewing
- 14.00-16.00** Scientific Session 3: Animal Models of Disease (Sigma)
Chair: Dr Rose Martiniello-Wilks (UTS, Sydney)
- 14.00-14.45 **Keynote speaker 3: Dr Tomoji Mashimo**, *Generation of Knockout Rats Using Zinc-Finger Nucleases* (Kyoto Uni., Japan)
- 14.45-15.00 O7: Professor Sue Fletcher, *Transient mouse models for the preclinical evaluation of therapeutic dystrophin exon skipping strategies* (CNND, University of WA)
- 15.00-15.15 O8: Dr Sharon Cunningham, *shRNA-mediated induction of severe hyperammonemia in the OTC-deficient Spfash mouse to assess the challenges of neonatal therapy with rAAV* (CMRI, Sydney)
- 15.15-15.30 O9: Dr James Chan, *Non-myeloablative transplantation of self-antigen expressing bone marrow prevents autoimmune disease by peripheral tolerance mechanism* (Monash University, Melbourne)
- 15.30-16.00** Afternoon Tea, Atrium Bio21 Institute
Trade Display & Poster Viewing
- 16.00-17.30** Poster Session 1 (see "Poster-Walkabout")
Chairs: Prof Steve Wilton and Dr Paul Gregorevic
Poster numbers 1-14
- 17.30-18.30** Welcome Drinks
Atrium, Bio21 Institute

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THURSDAY, 5th MAY, 2011

- 08.45-09.30** **Scientific Session 4: Gene Therapy 1**
Chair: Prof Ann Simpson (UTS, Sydney)
Bio21 Auditorium 1
- 08.45-09.00 O10: Dr Jim Vadolas, *Development and application of site-specific integration of functional genomic loci* (MCRI, Melbourne)
- 09.00-09.15 O11: Dr Matthias Klugmann, *Successful in vivo gene therapy for a white matter disease after adeno-associated virus vector delivery to oligodendrocytes* (UNSW, Sydney)
- 09.15-09.30 O12: Dr Kazuo Suzuki, *Sequence specific induction of HIV-1 transcriptional gene silencing targeting NF- κ B sequences by siRNA without any off-target effects of other NF- κ B driven genes* (St Vincent's AMR, Sydney)
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- 09.30-10.30** **Greg Johnson Memorial Oration**
Chair: Dr Jim Vadolas (MCRI, Melbourne)
Bio21 Auditorium 1
Keynote speaker 4: Prof Douglas Hilton, *Pathways regulating blood cell production* (Director WEHI, Melbourne)
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- 10.30-11.00** **Morning Tea, Atrium Bio21 Institute**
Trade Display
Poster Viewing
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- 11.00-12.30** **Scientific Session 5: Cell and Gene Therapy in the Clinic**
Chair: Prof Ian Alexander (CMRI, Sydney)
- 11.00-11.45 **Keynote Speaker 5: Prof Philippe Leboulch, *Conversion to transfusion independence with Hmga2 activation after lentiviral gene transfer for severe human β -thalassemia*** (Director, INSERM, France)
- 11.45-12.15 **Invited Speaker: Dr Dominic Wall, *Use of autologous peripheral blood T lymphocytes transduced with an anti LewisY chimeric receptor gene in patients with LewisY positive Multiple Myeloma, Acute Myeloid Leukaemia or high-risk Myelodysplastic Syndrome*** (Peter Mac, Melbourne)
- 12.15-13.00 **Invited Speaker: Prof David Kaye, *Gene therapy for heart failure*** (Baker IDI Institute, Melbourne)
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- 13.00-13.30** **Lunch, Atrium Bio21 Institute**
Trade Display
Poster Viewing

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THURSDAY, 5th MAY, 2011 (continued)

- 13.30-15.00** **Scientific Session 6: Non-coding RNA**
Chair: Prof Steve Wilton (CNND, University of WA)
- 13.30-14.15 **Keynote Speaker 6: Prof John Mattick**, *RNA at the epicentre of human development* (University of Queensland, Brisbane)
- 14.15-14.45 **Invited Speaker: Prof York Zhu**, *RNAi Therapeutics Screening for Gene Therapy* (CEO, Biomics Biotechnologies Co., Ltd China)
- 14.45-15.00 O13: Dr Nham Tran, *Co-Regulation of the Programmed Cell Death Protein 4 (Pdc4) by miR-21 and miR-499* (Young Investigator UTS, Sydney)
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- 15.00-15.30** **Afternoon Tea, Atrium Bio21 Institute**
Trade Display
Poster Viewing
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- 15.30-16.30** **Poster Session 2 (see “Poster-Walkabout”)**
Chair: Dr Gerry Both and Prof Sue Fletcher
Poster numbers 15-27
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- 16.30-18.00** **Australasian Gene Therapy Society Annual General Meeting**
Bio21 Auditorium 1

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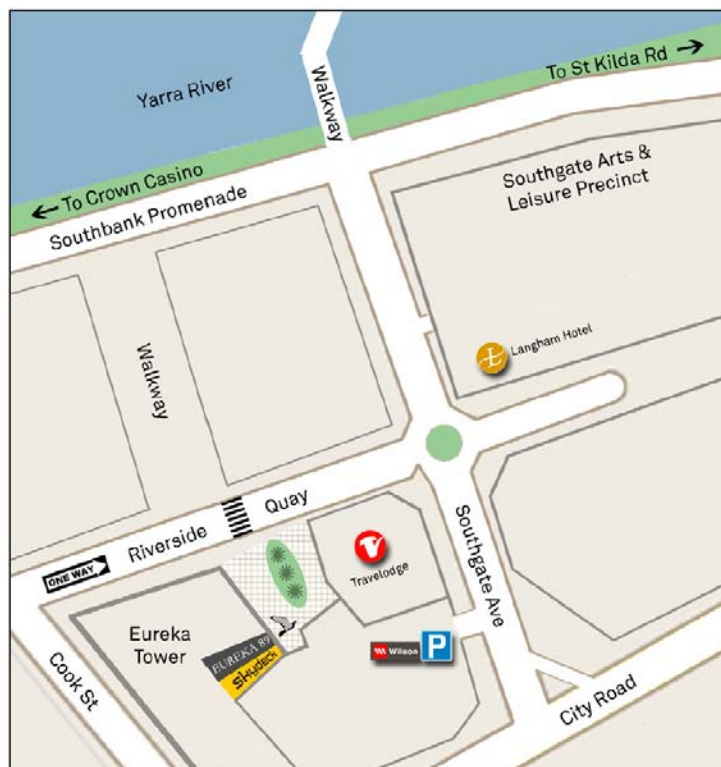
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THURSDAY, 5th MAY, 2011 (continued)

19.30- 23.30 Conference Dinner
EUREKA 89 Restaurant
Level 89 Eureka Tower
7 Riverside Quay Southbank
Victoria 3006 Australia
P: +613 9693 8889
F: +613 9693 8899
E: events@eureka89.com.au

EUREKA 89 Restaurant PARKING: Wilson Parking is our preferred partner for car parking and is located at two locations around the base of Eureka Tower. Wilson Car Parks can be accessed from the corner of City Road and Southgate Avenue or Riverside Quay, enter from City road into Southbank Boulevard and turn right into Riverside Quay. Eureka Level 89 visitor rates apply when exiting before 6.00am the following day. Monday – Friday (entry before 4.00pm): \$12.00
Monday – Friday (entry after 4.00pm): \$ 6.00
Weekends & Public Holidays: \$ 6.00

Please validate your parking ticket at the Eureka Level 89 exit when departing after 10.00pm.



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FRIDAY, 6th May, 2011

- 09.00-10.30 Scientific Session 7: Gene Knockdown (FSHD Global Research Foundation)**
Chair: Dr Paul Gregorevic, (Baker IDI Institute, Melbourne)
Bio21 Auditorium 1
- 09.00-09.45 **Keynote speaker 7: Prof Alexandra Belayew, *Suppression of DUX4 or DUX4c expression by antisense strategies in a therapeutic approach for FSHD*** (Uni. Mons, Belgium)
- 09.45-10.15 **Invited Speaker: Prof Melanie Ehrlich, *Finding targets for FSH dystrophy therapy*** (Tulane University, USA)
- 10.15-10.30 O14: Dr Michael Gantier, *AntagomiR-mediated inhibition of inflammation* (Young Investigator, Monash, Melbourne)
- 10.30-11.00 Morning Tea, Atrium Bio21 Institute**
Trade Display
Poster Viewing
- 11.00-13.00 Scientific Session 8: Gene Therapy 2**
Chair: Dr Grant Logan (CMRI, Sydney)
- 11.00-11.45 **Keynote speaker 8: Dr Dirk Grimm, *Synthetic AAV vectors as tools for safe shRNA expression & targeted miRNA regulation*** (Germany)
- 11.45-12.00 O15: Dr Paul Gregorevic, *The potential and limitations of manipulating TGF- β signalling to treat muscle wasting* (Baker IDI Institute, Melbourne)
- 12.00-12.15 O16: Cindy Kok, *Transfer of AAV-specific maternal antibodies in breast milk impedes vector delivery to juvenile mice* (Student CMRI, Sydney)
- 12.15-12.30 O17: Henna Kuusisto, *Enhanced nuclear transport in transformed cells; prospects for anti-cancer gene therapy?* (Student Monash, Melbourne)
- 12.30-12.45 O18: Allison Dane, *Investigating transgene expression across the hepatic lobule after rAAV mediated gene delivery* (Student CMRI, Sydney)
- 12.45-13.00 O19: Dr Rob Kapsa, *Microstructured hybrid conducting polymer platforms for skeletal muscle* (University of Melbourne)
- 13.00-14.00 Lunch, Atrium Bio21 Institute**
Trade Display
Remove posters

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FRIDAY, 6th May, 2011 (continued)

- 14.00-16.00 Scientific Session 9: Gene Manipulation**
Chair: Dr Rose Martiniello-Wilks (UTS, Sydney)
- 14.00-14.45 **Keynote Speaker 9: Prof Dave Russell**, *Gene Targeting with Adeno-Associated Virus Vectors* (Washington University, USA)
- 14.45-15.15 O20: Dr Albert Mellick, *MicroRNAs as Tools to Target Bone Marrow Mediated Tumour Angiogenesis* (Griffith University, Brisbane)
- 15.15-15.30 O21: Dr Claus Hallwirth, *Generation and analysis of a highly complex γ -retroviral vector integration site dataset from human CD34+ cells using Illumina next-generation sequencing* (Young Investigator CMRI, Sydney)
- 15.30-16.00 O22: Professor Steve Wilton, *Personalised Genetic Therapies for Neuromuscular Diseases* (CNND, University of WA)

16.00-16.30 Panos Ioannou Young Investigator Award and Student Prizes

Closing remarks by the President

Note: “Poster-Walkabout”

In the poster sessions, there will be a “poster walk-about”, where 2 chairs will introduce each poster and presenting author, who will then give a 2 minute overview of their work (imagine you are in an elevator for 2 minutes with someone important) followed by 3 minutes of question and answers to provide more detail. Timing will be strictly enforced.

Note: All oral powerpoint presentations should be provided to the Audio Visual Operator 30 minutes before activities begin each day (8am-8.30am)

Bio21 Institute

The University of Melbourne,
30 Flemington Rd (near corner of Park Dve),
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Melways ref: 2A, K7



Transport: From the CBD, take the 59 tram (along Elizabeth St) or take the 55 tram (along William St) to Park Dve. Tram Stop 15 is closest to Bio21.

Parking: No public parking is available on site at Bio21. Limited street and meter parking is available in the surrounding streets. The closest all day public parking is at University Square Carpark (enter Berkeley St or Bouverie St, Carlton), which is approximately a 10 min walk to Bio21). Alternative public parking is at the Royal Children's Hospital on Flemington Road (access from Gatehouse Road), the Royal Women's Hospital (access from Flemington Road) or the Royal Melbourne Hospital car park (off Royal Pde).

Airport: 25 minutes from Melbourne International Airport via freeway.