33rd NSW STEM CELL NETWORK WORKSHOP

TRANSLATING CELL THERAPY DISCOVERIES INTO THE CLINIC

THE CULLEN ROOM, UNIVERSITY OF SYDNEY

Tuesday, 21st May, 2024
9.00am to 5.00pm

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The NSW Stem Cell Network gratefully acknowledges the support of Diabetes NSW & ACT and CCRM Australia.

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WELCOME

The NSW Stem Cell Network is a not-for-profit organisation that started in 2002 as a professional community aimed at bringing together the scientific, medical, business, ethics, law, and higher education sectors, to promote innovation and growth in Australian research and healthcare. The NSW Stem Cell Network is comprised of a Board of professionals with backgrounds in medicine, translational and medical research, and business. It has a membership base of over 1000 members from academia, industry, and public communities. To date, we have hosted 32 workshops encompassing the areas of stem cells and regenerative medicine and cell and gene therapies, provided 4 training courses around the use of pluripotent stem cells, and have compiled 9 Snapshots of Regenerative Medicine Companies in Australia.

This year, the NSW Stem Cell Network is working in cooperation with CCRM Australia to provide commercialisation and training services to the SMART CRC initiative, supporting the acceleration of regenerative therapies and their adoption in Australia.

In the first of a series of workshops, this workshop – Translating Cell Therapy Discoveries into the Clinic – has been put together to help guide researchers through some of the necessary steps and key challenges facing the clinical development of cell therapies.

Alongside our impressive list of expert speakers, we are excited to introduce two panel sessions in this program to help promote discussion and interaction amongst experts and the audience. In round table session 1 – *Process Development 101: How to Develop a Robust Cell Therapy Manufacturing Process* – we will discuss the challenges faced by scientists in moving from laboratory-based research into a clinical manufacturing process that meets the requirements for cell therapy clinical trials. This includes issues related to scalability, reproducibility, and establishment of robust quality control measures. We will also touch on considerations for designing nonclinical studies that will lay the groundwork for progressing to first in-human and subsequent clinical trials. The session will be formatted as a Q&A session with the discussion lead by Chair, Dr. Sharon Sagnella.

In round table 2 – *When to Engage with Consultants, Contract Research Organisations (CROs), and Regulatory* – an expert panel will discuss how to select and work with consultants, CROs, and regulatory bodies towards the development of a cell therapy for use in the clinic. This session will help participants identify CROs and consultants that meet their needs and what questions to ask when approaching CROs, consultants, and regulatory bodies. It will help participants understand the function of these different bodies in the clinical cell therapy development pipeline and when the right time to approach them is. This session will also be formatted as a Q&A session with the discussion lead by Chair, Prof. John Rasko.

This workshop would not have been made possible without the support of our commercial sponsors ThermoFisher Scientific, Pacific Laboratory Products, Laftech, Children's Medical Research Institute, Miltenyi Biotec, Bio Scientific, Capella Science, StemCore, BioTools, Millennium Science, Bio-Strategy and Inventia; and our supporters Diabetes NSW, CCRM Australia, and the SMART CRC initiative. We encourage all attendees to visit the sponsor booths during the breaks.

We hope you enjoy the workshop and continue to support the NSW Stem Cell Network at future events.

Deb Rooz
Manager
NSW Stem Cell Network

Dr Rachel Shparberg
Chair
NSW Stem Cell Network

We acknowledge the Gadigal people of the Eora Nation, who are the traditional owners of the land on which this workshop takes place.

PROGRAM

9:00am	Registration Opens
9.30am	**Dr Rachel Shparberg - NSW Stem Cell Network** Welcome
Session 1	**Process Development** **Chair: Dr. Sharon Sagnella (Royal Prince Alfred Hospital)**
9.40am	**Dr Janet Macpherson (Cytiva)** *Navigating process development*
10.10am	**Dr Leighton Clancy (NSW Health)** *Navigating the path to clinical translation: process development considerations for cell therapies*
10.40am	Morning Tea
11.00am	**Round Table 1: *Process Development 101: How to Develop a Robust Cell Therapy Manufacturing Process*** **Chair:** Dr Sharon Sagnella **Panel:** Dr Leighton Clancy (NSW Health), Dr Janet Macpherson (Cytiva), Dr Andrew Prowse (StemCore), Dr Chih Wei Teng (CCRM Australia)
Session 2	**Quality Assurance** **Chair: Dr Janet Macpherson (Cytiva)**
11.50am	**Jo Karra (Sydney Children's Hospital Network)** *Journey towards GMP and establishing QMS*
12.20pm	**Sharan Gopal (Royal Prince Alfred Hospital)** *Quality management in cell and molecular therapies*
12.50pm	Lunch
Session 3	**Regulatory and Ethics** **Chair: Sara Attinger (University of Sydney)**
1.40pm	**Dr Gabrielle O'Sullivan (Royal Prince Alfred Hospital)** *Regulatory considerations in translating cell therapy discoveries into the clinic*
2.10pm	**Prof Dianne Nicol (University of Tasmania)** *Ethical, legal, and social issues in translating cell therapy discovery into the clinic*
2.40pm	Afternoon Tea
Session 4	**Clinical Trials** **Chair: Prof John Rasko AO (Royal Prince Alfred Hospital, University of Sydney, Centenary Institute)**
3.00pm	**Dr Aimei Lee (Royal Prince Alfred Hospital)** *Conducting clinical trials: site logistics*
3.30pm	**Greg Plunkett (Accelagen)** *Making the jump into the clinic!*
4.00pm	**Round Table 2: *When to Engage with Consultants, CROs and Regulatory*** **Chair:** Prof John Rasko AO (Royal Prince Alfred Hospital, University of Sydney, Centenary Institute) **Panel:** Sara Attinger (University of Sydney), Greg Plunkett (Accelagen), Helen Wray (Skin2Neuron), Dr Avril Fortuin (Advanced and Biological Therapies, TGA)
4.50pm	Workshop Close

SPEAKER PROFILE

DR JANET MACPHERSON

Business Development Manager
Enterprise Solutions Cell & Gene Therapy
Cytiva

ABSTRACT

Navigating Process Development

This presentation will address the top 4 Process Development challenges for cell therapy manufacturing:

- Addressing open and manual processes
- Tackling resource limitations
- Creating a robust design
- Navigating regulatory requirements

BIOGRAPHY

Dr Janet Macpherson joined Cytiva 5 years ago and is currently Business Development Manager Enterprise Solutions Cell & Gene Therapy at Cytiva, responsible for Cell Therapy manufacturing solutions comprising hardware, documentation packages, digital, training, and process development services supporting the regional teams across Asia Pacific. Janet has over 30 years' experience in research and product development in the Cell & Gene Therapy sector and has held leadership roles in both industry (Johnson & Johnson Research) and healthcare/academia (Sydney Local Health District) where she was responsible for cross-functional cell and gene therapy product development teams, cell therapy process development and closed system personalised manufacturing.

Janet was awarded a PhD from the University of NSW for studies on Human mast cells and immune mediators. She is experienced in clinical trial development and implementation including pre-clinical, clinical, CMC data packages, development of study plans and Annual and final study reports and data interpretation. She has managed and executed clinical trials from both the Sponsor and Investigator site perspective.

Janet is an active member of the International Society for Cell and Gene Therapy (ISCT) and has served on the Global Executive committee as regional Vice-President for Australia and New Zealand and is a member of the ISCT Asia Industry committee.

SPEAKER PROFILE

DR LEIGHTON CLANCY

Operations Manager
Sydney Cell and Gene Therapy
Western Sydney Local Health District

ABSTRACT

Navigating the path to clinical translation: process development considerations for cell therapies

Translating cell therapies, from research to clinical application, presents significant challenges in process development and GMP manufacturing. This talk will explore key considerations and strategies for successfully transitioning from an R&D method to a GMP-compliant process, drawing from our experience in developing a T cell therapy manufacturing process.

BIOGRAPHY

Dr Leighton Clancy completed his BSc (Hons) and PhD at the University of New South Wales in medical virology. In 2006 he joined the Sydney Cellular Therapies Laboratory at Westmead Hospital and was involved in establishing manufacturing capabilities for donor-derived and "off the shelf" T cells in multiple early phase trials. He was responsible for establishing a bank of virus specific T cells targeting cytomegalovirus, Epstein-Barr virus and adenovirus that has been accessed by transplant recipients in multiple centres in Australia and New Zealand. Dr Clancy is currently the Operations Manager for Sydney Cell and Gene Therapy at Westmead Hospital.

SPEAKER PROFILE

JO KARRA

Quality Manager
Viral Vector Manufacturing Facility
Gene Therapy Research Unit,
Sydney Children's Hospital Network

ABSTRACT

Journey towards GMP and establishing QMS

GMP is critical for ensuring the safety and efficacy of the manufactured products. The aim of this presentation is to provide guidance for research/academic facilities on how to establish QMS, implement and comply with cGMP standards. It will provide an understanding of critical documents that need to be established, requirements of a manufacturing facility, personnel and the product manufacturing process . It further provides insights on validation requirements and the Quality control and assurance aspects that need to be considered to comply with cGMP standards. The presentation further illustrates the challenges encountered by VVMF on their journey towards GMP.

BIOGRAPHY

Jo Karra has over 20 years' experience in the Advanced Therapy Medicinal Products (ATMP), Biologics and Pharmaceutical industries with extensive systems knowledge across Quality and Operations Management, and is experienced in working within multinational pharma, CDMO's and startup companies.

She is currently working as Quality Manager at Viral Vector Manufacturing Facility operating within SCHN. She is leading the establishment of QMS and developing operational and facility procedures that would enable VVMF to gain TGA licence and be Australia's first viral vector manufacturing facility with regulatory approval. She ensures that a quality culture, patient safety first mindset is developed within the team through training and providing common understanding.

Previous roles involved Quality Control Unit Manager at Aegros and laboratory leadership roles for CSL, with accountability for establishment of new lab, lab procedures, method validations, equipment qualification and sample management.

SPEAKER PROFILE

SHARAN GOPAL

Quality Officer
Royal Prince Alfred Hospital Cell and Molecular Therapies

ABSTRACT

Quality Managemement in Cell and Molecular Therapies

How systems can be utilised to implement and maintain Quality Management according to regulations and standards in Cell Therapies, including why Change Control Plans are necessary for transitioning systems.

BIOGRAPHY

In the role of Quality Officer for almost three years, I have been responsible for maintaining and enhancing quality standards across all cellular therapy operations at RPAH from collection till infusion and disposal of commercial and clinical trial products. My position involves ensuring compliance with various regulatory bodies, including The Therapeutic Goods Administration (TGA), The Foundation for the Accreditation of Cellular Therapy (FACT), and The Office of the Gene Technology Regulator (OGTR). This also comprises adherence to stringent standards such as ISO15189, Good Manufacturing Practice (GMP), Good Laboratory Practice (GLP), Good Clinical Practice (GCP), and the National Safety and Quality Health Service (NSQHS) framework for safeguarding patient outcomes and institutional credibility.

Before this role, I attained over nine years of experience as a Medical Laboratory Scientist within NSW Health Pathology and NSW Tissue Bank where I dedicated myself to ensuring the accuracy and integrity of diagnostic testing and tissue retrieval, processing and storing. I honed my skills in analysing specimens, interpreting results, and maintaining equipment and facilities to aid in patient diagnoses and treatment plans while complying to the National Association of Testing Authorities (NATA) and TGA.

Throughout my career, I've navigated the complexities of regulatory requirements with unwavering commitment and strived to foster a culture of continuous improvement and excellence within my respective domains. My dedication to upholding the highest standards of quality and safety has been driven by scientific experience in the hospital space and a profound sense of responsibility to the patients we serve and the broader healthcare community.

In this workshop, I am happy to meaningfully contribute to the advancement of healthcare delivery and patient care through influencing others to understand and value quality assurance in health services and research.

SPEAKER PROFILE

DR GABRIELLE O'SULLIVAN

Executive Officer
Royal Prince Alfred Hospital Institutional Biosafety Committee
Research Ethics and Governance Office
Royal Prince Alfred Hospital

ABSTRACT

Regulatory considerations in translating cell therapy discoveries into the clinic

This presentation will cover broad regulatory aspects for consideration when planning to translate cell therapy research discoveries into the clinic. It will also outline specific regulatory requirements for cell therapies that involve minimal and more than minimal manipulation of cells, with a focus on gene modified cell therapies such as Chimeric Antigen Receptor (CAR) cell therapies. Examples of approvals, translation partners, and regulatory relevant processes and decisions will be given. The perspectives of primary developers, clinical trials, sponsors, and hospitals will be considered.

BIOGRAPHY

Dr Gabrielle O'Sullivan is the Executive Officer of Royal Prince Alfred Hospital Institutional Biosafety Committee and has extensive experience in the regulation and risk assessment of gene technology, particularly in the contexts of biosafety, biomedical research, clinical trials, and cell and gene therapies. She is a member of the Australian Government Department of Health Gene Technology Technical Advisory Committee (GTTAC) and Gene Technology Ethics and Community Consultative Committee (GTECCC), co-chair of the ANZ Legal and Regulatory Affairs Committee of the International Society Cell & Gene Therapy (ISCT), and a member of the Ausbiotech & Medicines Australia Cell & Gene Therapy Catalyst Expert Working Group on policy and advocacy.

SPEAKER PROFILE

PROF DIANNE NICOL

Distinguished Professor Emerita
University of Tasmania

ABSTRACT

Ethical, legal and social issues in translating cell therapy discovery into the clinic

It has long been recognised that the ethical, legal and social implications (ELSI) of genomic and stem cell research require particular attention. In the early years, much attention was focused on concerns about use of human embryos for research and about germline transmission of genomic modifications. Although these issues still remain relevant (as do traditional regulatory concerns around safety, efficacy and utility), ELSI discussions in this area increasingly focused on such matters as public trust, equity and justice – recognising the need for diversity in datasets, equitable access and benefit sharing. This presentation will start with an overview of the regulatory environment for cell therapy translation. The presentation will then focus on three key themes exploring how public trust, equity and justice could be given greater prominence: new models for consent; intellectual property and commercialisation; transparency and accountability in regulatory decision making.

BIOGRAPHY

In 2021, Dianne Nicol retired from her positions as Distinguished Professor of Law and Director of the Centre for Law and Genetics at the University of Tasmania in Australia. She continues her involvement in academia as a Distinguished Professor Emerita. Dianne has a background in science as well as law, with a PhD in cell biology. She was elected as a fellow of the Australian Academy of Law in 2016 and the Australian Academy of Health and Medical Sciences in 2020, and is the current chair of the NMRC Embryo Research Licensing Committee. Dianne's current research focuses primarily on the ethical, legal, and social implications of personalized medicine, genomic data sharing, stem cell technology, biobanking, genome editing, and other emerging health technologies, together with commercialisation of biotechnology and patenting of biotechnological inventions. She recently a completed a citizen engagement project, The Australian Citizens' Jury on Genome Editing.

SPEAKER PROFILE

DR AIMEI LEE

Clinical Trials Manager
Cell & Molecular Therapies
Royal Prince Alfred Hospital

ABSTRACT

Conducting Clinical Trials: Site Logistics

Clinical trials are a necessary step in bringing new therapies to the market. We often see and hear stories in the media about a patient participating in a clinical trial and receiving a new therapy that could revolutionise the way a particular disease is treated or managed. But have you ever thought about how a clinical trial is conducted at a hospital? This presentation will outline the site logistics and highlight some of the work that clinical trial coordinators do in order to run a clinical trial within a public hospital.

BIOGRAPHY

Dr Aimei Lee is the Clinical Trials Manager for the department of Cell & Molecular Therapies at Royal Prince Alfred Hospital (RPAH).

Aimei commenced working in clinical trials as a Technical Officer in 2012 for the A.W. Morrow Gastroenterology and Liver Centre at RPAH, while completing her PhD in liver disease at the University of Sydney. The following year she moved into the role of Clinical Trial Coordinator and has worked on gastroenterology and hepatology clinical trials at RPAH and Liverpool Hospital.

Since joining the Cell & Molecular Therapies department in 2019, Aimei has worked on phase 1, 2, and 3 clinical trials using advanced therapy medicinal products. She manages a team of research nurses and is responsible for managing the implementation and progress of clinical trials from feasibility through to completion.

SPEAKER PROFILE

GREG PLUNKETT

CEO Accelagen Pty Ltd

ABSTRACT

Making the jump into the clinic!

The commencement of human clinical trials is a monumental milestone in the development of new therapies. In order to maximise the outcomes whilst maintaining integrity and utility of the data is crucial, so careful planning on the study design is important for both sites and sponsors. Asking the right questions and collecting the right information will evolve the pathway forward. In this presentation, we will review important factors in the design of a new clinical study, whether it be a first in human trial or a pivotal efficacy evaluation. Choosing the right patients, the most appropriate endpoints and managing data will set up the product to future success.

BIOGRAPHY

Greg Plunkett is the founder and CEO of Accelagen Pty Ltd, a Melbourne headquartered regulatory affairs and clinical development CRO. The company was founded in 2010, and has been working with local and overseas companies develop new and exciting therapies that have progressed through clinical trials and achieved regulatory approvals. The company's mission is to make a difference to the future of human health, and through their work, the benefits seen to many Australian patients has been inspirational. Working across large pharma through to start up biotechs has allowed Greg to see the evolution of new products from concept to commercialisation.

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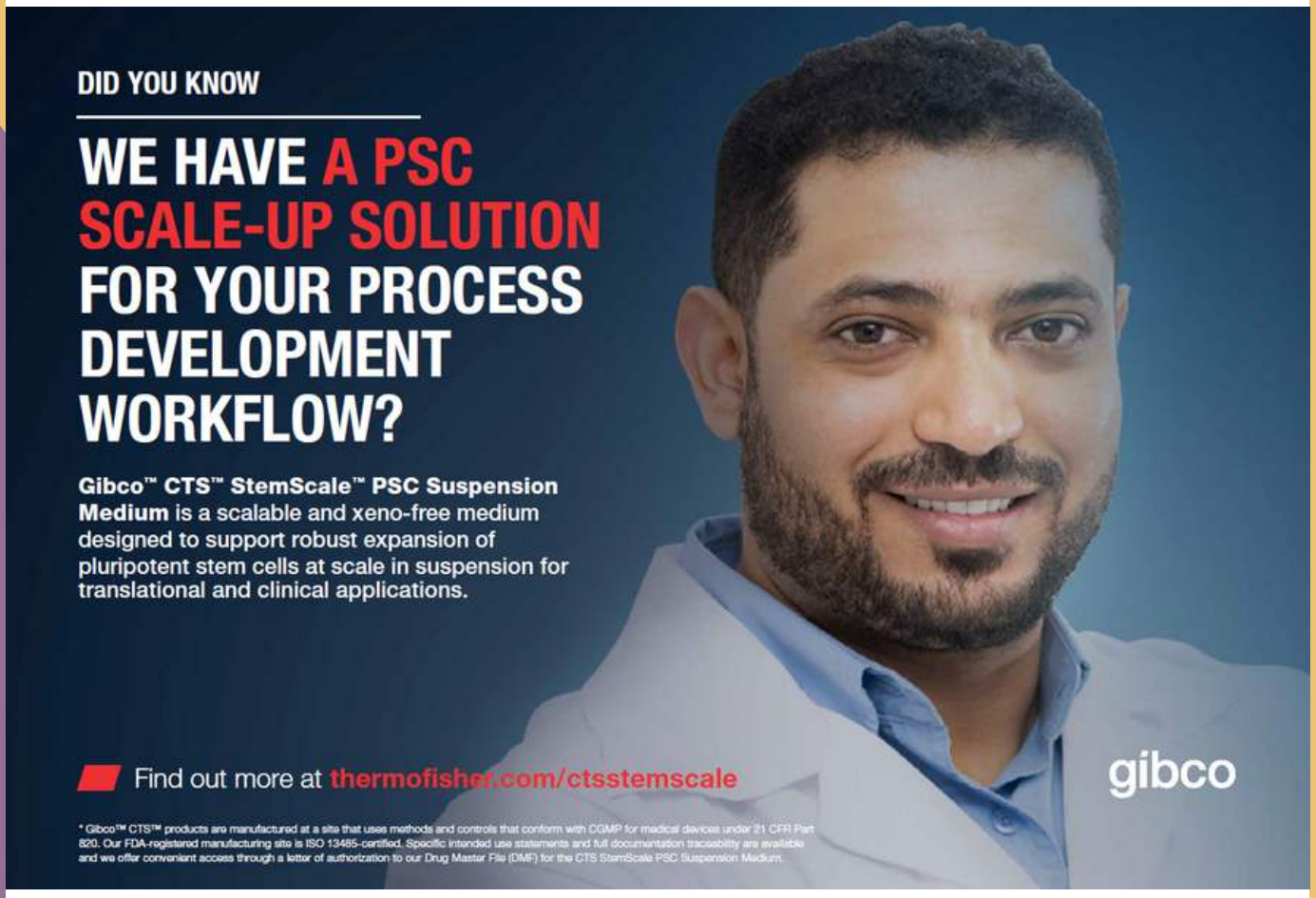

JOIN THE NSW STEM CELL NETWORK

The NSW Stem Cell Network is a professional community with an interest in all forms of stem cells. Our all inclusive free membership makes this network unique in consisting of not only researchers and practitioners, but members of the public, industry and government bodies. Our aim is to ensure effective communication between diverse sectors for the advancement of stem cell research. As a member, you will receive invitations to upcoming network and external stem cell-related events.

Sign up at www.stemcellnetwork.org.au

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