31st NSW STEM CELL NETWORK WORKSHOP

CELL THERAPY:-
BENCH TO BEDSIDE TO REIMBURSEMENT

AERIAL UTS FUNCTION CENTRE

Monday, 8th May 2023
9.00am to 5.00pm

NSW StemCell NETWORK

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The NSW Stem Cell Network gratefully acknowledges the support of Diabetes NSW & ACT, and the Aerial University of Technology Sydney Function Centre.
We wish to acknowledge the involvement and efforts of Dr Anai Gonzalez-Cordero, Dr Janet Macpherson, Dr Heather Main, and Dr Margret Schuller in creating this workshop.

WELCOME

Cell therapy, the transfer of cellular material into a patient for the purpose of repairing or regenerating tissue, has gained significant interest over recent years across a range of medical fields. Like all new therapies, a variety of challenges must be overcome throughout the stages of translation and manufacture through to the product approval, 1) to ensure that a safe, efficacious, and ethical product is delivered to patients and 2) to ensure compliance to regulatory frameworks.

This workshop has been put together to provide an overview of the processes involved in translating cell therapy research to the clinic, whilst at the same time, ensuring the industrial and commercial requirements for successful movement of therapies to market approval, reimbursement and standard of care are met.

The first session of the day – 'Towards Good Manufacturing Practices (GMP) Batches' – will be dedicated to understanding what it takes to prepare a GMP product. Following an introduction on GMP for cell-based therapies by Dr Heather Main, Fergus O'Connell will outline the fundamental analytical considerations required throughout product development through to their release. Dr Melanie Domingues and Dr Hwee Ing Ng will then describe what it takes to run commercial and research GMP facilities for cell therapies, respectively. Once a cell therapy is ready for market, there must be regulations in place to ensure the safe and ethical distribution to patients, which Dr Tessa Sherry will discuss to conclude session one.

After morning tea, we will move onto the second session, which covers 'Hospital Implementation' of cell therapies. In this session, Dr Sharon Sagnella will discuss how clinical trials are implemented, followed by Dr Janet Macpherson who will present on GMP compliance for cell therapies in the hospital setting. Challenges arising at the state legislative level will then be discussed by Dr Olivia Hibbitt and Dr Filimon Haile.

Following lunch, session 3 – 'Commercial Suitability' – will begin with Anne Maree Englund providing insights into the industry perspective on health technology assessment (HTA) for cell and gene therapies. David Kneen will then discuss the role of engineers in driving the commercial suitability of cell therapies, followed by a discussion from Dr Nick Simpson on HTA processes and negotiations for cell-based medicines.

In session 4 – 'Market Approval for Cell Therapy' – Dr Kilian Kelly will present findings on the development of Class IV biological cell therapies. Professor Minghao Zheng and Dr Silviu Itescu will then discuss market approval and implementation of cell therapies in Australia.

We anticipate this workshop to be an exciting and unique opportunity for Australian and international researchers, industry representatives, and clinicians to meet and share their latest findings and research, and to help bridge the gap between research and patient care.

This workshop would not have been possible without the support of our steering committee, the Aerial University of Technology Sydney Function Centre, and our commercial sponsors BioTools, Cell Therapies, Cytiva, Evident Scientific, Hoya, Miltenyi Biotec, NSW Ministry of Health, Q-Gen Cell Therapeutics, and Therapeutic Innovation. We encourage all attendees to visit their booths during the program 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

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PROGRAM

9:00am	Registration Opens
9.30am	**Dr. Rachel Shparberg - NSW Stem Cell Network** Welcome
Session 1	**Towards GMP Batches** **Chair: Prof. Nick Di Girolamo (University of NSW)**
9.40am	**Dr. Heather Main (ATMP Sweden)** *Introduction to GMP for cell based medicines*
10.00am	**Fergus O'Connell (Eurofins Scientific)** *Microbiological analytics for cell-based therapies – releasing with confidence*
10.20am	**Dr. Melanie Domingues (Cell Therapies Pty. Ltd.)** *What it takes to run a GMP facility for cell therapies - commercial*
10.40am	**Dr. Hwee Ing Ng (Q-Gen Cell Therapeutics)** *What it takes to run a GMP facility for cell therapies - research*
11.00am	**Dr. Tessa Sherry (Therapeutics Goods Administration)** *Regulation of cell therapies in Australia*
11.20am	Morning Tea
Session 2	**Hospital Implementation** **Chair: Dr. Anai Gonzalez-Cordero (The University of Sydney)**
11.50am	**Dr. Sharon Sagnella (Royal Prince Alfred Hospital)** *Implementation of advanced therapeutic medicinal product clinical trials in a public hospital*
12.10pm	**Dr. Janet Macpherson (Cytiva)** *Closing up your process for cGMP compliance*
12.30pm	**Dr. Olivia Hibbitt (NSW Ministry of Health), Dr. Filimon Haile (Vic. Department of Health)** *State challenges in implementation, reimbursement and follow up for cell therapy medicines*
12.50pm	Lunch
Session 3	**Commercial Suitability** **Chair: A/Prof. James Chong (The University of Sydney)**
1.50pm	**Anne Maree Englund (Medicines Australia)** *Industry perspective on health technology assessment for cell and gene therapies*
2.10pm	**David Kneen (Celleo Biotech)** *The role of engineers in driving commercial suitability of cell-based medicines*
2.30pm	**Dr. Nick Simpson (Commonwealth Department of Health & Aged Care)** *Health technology assessments and negotiations for cell-based medicines*
2.50pm	Afternoon Tea
Session 4	**Market Approval for Cell Therapy** **Chair: Prof. Bernie Tuch (NSW Stem Cell Network)**
3.20pm	**Dr. Kilian Kelly (Cynata)** *Development of Class IV biological cell therapies*
3.40pm	**Prof. Minghao Zheng (Orthocell)** *Market approval and implementation of a cell therapy*
4.00pm	**Dr. Silviu Itescu (Mesoblast)** *Bringing MSC products for FDA approval*
4.20pm	**Panel session**
4.45pm	Workshop Close

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SPEAKER PROFILE

DR. HEATHER MAIN

Project Manager ATMP Sweden
Co-founder HOYA Consulting, Stockholm, Sweden

ABSTRACT

Introduction to GMP for cell-based medicines

Do you have a xeno free, defined, robust, scalable protocol for producing your research grade cell therapy?...So did we....4 years later we froze down our clinical batch. Adapting your cell therapy to GMP is a world of 'chemistry, manufacturing and controls' that most academics have never been exposed to. Quality in manufacture requires establishment of documentation to define the product prior to manufacture, safety, identity and potency, as well ask risk assessment of the process and product. This presentation summarises this for cell based medicines as well as commercial considerations needed to take cellular therapies not just bench to bedside, but to reimbursement. From raw materials compliance to personnel availability and qualification, facility operations, equipment qualification, counting validation, process locking and validation, the challenges along the way lead to a better product through better understanding and thus control of the process. I also touch on the required competencies for clinical translation of research cellular therapies, including GMP, cell-based medicine and pharma experts. Working together to create products not only suitable for an academic Phase I clinical trial but to commercialisation is necessary for the patient access promised by our field.

BIOGRAPHY

Heather Main, PhD, has worked with pluripotent stem cells since 2003 since studying Molecular Biology and Biochemisty at The University of Adelaide, then going on to complete her PhD at Karolinska Institutet, Stockholm, Sweden. Heather had the opportunity to be a visiting scientist at A*Star/Singapore, CMRI/Sydney and NIH/Maryland and then completed a post doctoral position at the University of Sydney. During her postdoc Heather worked as the Manager of the NSW Stem Cell Network, running the 18th and 19th workshops. Heather moved into commercial application of hPSC with the company Genea Biocells and moved with the company from Sydney, Australia to San Diego, California. Upon returning to Sweden, in 2018 Heather performed intertwining functions translating a hPSC-RPE protocol to hands on GMP manufacture and product release and as a Project Manager developing Sweden's National and International cell and gene therapy pharmaceutical network 'ATMP Sweden'. Heather continues in this ATMP Sweden role and now runs the consulting company HOYA, specializing in services for development of clinically and commercially suitable hPSC derived cellular therapies.

SPEAKER PROFILE

FERGUS O'CONNELL

Head of Quality - Eurofins
BioPharma Testing, Australia/New Zealand

ABSTRACT

Microbiological analytics for cell-based therapies – releasing with confidence

Microbiological safety is an essential quality attribute of cell-based therapies regulated as biological products.
Sterility, endotoxin and mycoplasma testing play an important part, amongst a suite of tests, helping to reliably obtain adequate product quality and yield. They help to gather data related to process and product characterization while supporting assessments of any changes and/or excursions experienced during manufacturing.
This presentation will outline the fundamental considerations for these tests along with their place within product development, in-process control, and finished product release testing.

BIOGRAPHY

Fergus has over 25 years of QA experience in the pharmaceutical industry in the areas of sterile manufacturing, inhalations, solid dose manufacturing, & analytical testing.
His various roles in quality have included validation management, production support, complaint management and that of a European QP (Qualified Person) responsible for the release of finished products to markets in the USA, Europe, and the Asia-Pacific region.
He joined Eurofins Laboratories in 2011 as a QA Manager and is currently Head of Quality for the Eurofins BioPharma Testing Division in Australia and New Zealand.

SPEAKER PROFILE

DR. MELANIE DOMINGUES

Cell Therapy Specialist,
Cell Therapies Pty. Ltd.

ABSTRACT

What it takes to run a GMP facility for cell therapies – commercial

Running a GMP facility for cell therapies requires a high level of commitment to quality expertise, and adherence to GMP guidelines. It is essential to have substantial investment both qualitatively and quantitatively in infrastructure, equipment and staff which must be supported by a robust and effective management system to ensure execution of the manufacturing process at the highest quality standards.

Here, I will take you through Cell Therapies' journey with our experience in manufacturing Kymriah™, supplying the domestic market. The opportunity, challenges, and learnings of performing a technology transfer through to commercial manufacturing from the perspective of recruitment, training, supply chain and more.

BIOGRAPHY

Dr. Melanie Domingues is a Cell Therapy Specialist at Cell Therapies.

Melanie has over 10 years' international experience in cancer research and stem cell biology.

Within Cell Therapies, Melanie's role is to lead, manage and provide technical expertise on projects. Melanie is involved in several projects from process development to GMP manufacture of cell-based therapies for clinical and commercial supply.

Cell Therapies is a leading contract manufacturer and distributor of cellular therapies around the globe that specialises in developing world-class solutions to deliver cellular therapies for clinical trial and approved therapeutic uses.

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SPEAKER PROFILE

DR. HWEE ING NG

Senior Production Officer,
Q-Gen Cell Therapeutics,
QIMR Berghofer Medical Research Institute

ABSTRACT

Working with a contract development and manufacturing organisation

Stem cells, as a collective, are one of the most promising cell therapeutics with the potential for regeneration and enhancement of the body repair mechanism, for a wide range of disease targets, from hematologic malignancies, spinal injuries, to skin grafts for burn victims, providing new approaches to incurable diseases. Though research in the stem cell field is extensive, translation to clinical application has been challenging.

Here, we describe experiences at Q-Gen Cell Therapeutics, a contract development and manufacturing organisation (CDMO), situated within Queensland Institute Medical Research Berghofer (QIMRB), when translating a promising research idea to a clinical product. We will be discussing the common challenges faced by researchers when working with a CDMO, what you can expect and what pre-planning could be done to help accelerate compliance and timelines.

Being a facility within the QIMRB, we have a unique environment to collaborate and advise multiple researchers and industry led trials. This presentation will aim to provide some context to the common gaps between research and clinical manufacturing, to ultimately increase the exposure of stem cell applications in clinical context.

BIOGRAPHY

Dr. Hwee Ing (Jess) Ng has worked in the medical device and pharmaceutical sector extensively, in both academia and industry. In her current role as Senior Production Officer at Q-Gen Cell Therapeutics in QIMR Berghofer, she has been involved in translating multiple projects from research to the GMP facility.

Prior to this, she worked in a medical device startup company, where she developed and established fit for purpose analytical testing. She has also worked collaboratively with many departments across various functional teams and projects.

Her unique multidisciplinary background stems from her education, where she completed her Bachelors of Science majoring in Immunology and Infectious Disease (Honours I), followed by her PhD in Biotechnology and Systems Vaccinology in the Australian Institute of Bioengineering and Nanotechnology (AIBN) in The University of Queensland (UQ).

She is also a graduate of the Advanced GMP course from the Centre of Biopharmaceutical Excellence (CBE) and the Bridge Program from Queensland University Technology (QUT).

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SPEAKER PROFILE

DR. TESSA SHERRY
Senior Scientific Evaluator,
Therapeutic Goods Administration

ABSTRACT
Regulation of cell therapies in Australia

Dr. Sherry from Therapeutic Goods Administration (TGA) will present on the current state of regulation of cell therapies in Australia, including evaluation under the biologicals framework and the clinical trial pathways.

BIOGRAPHY
Dr. Tessa Sherry is a Senior Scientific Evaluator in the Biological Science Section at TGA. The Biological Science Section regulates blood and biologicals, and evaluates the quality of biological medicines, gene therapy products and infectious disease safety of therapeutic goods. Tessa gained her PhD in genetics and biochemistry from Monash University, and has been working in the Biological Science Section and TGA for 3 years.

SPEAKER PROFILE

DR. SHARON SAGNELLA

Research and Development Manager,
Cell & Molecular Therapies,
Royal Prince Alfred Hospital

ABSTRACT

Implementation of advanced therapeutic medicinal product clinical trials in a public hospital: the Department of Cell & Molecular Therapies

Sydney Local Health District (SLHD) has developed infrastructure comprised of highly skilled personnel operating a specialised and GMP facility, the Department of Cell & Molecular Therapies (CMT) at Royal Prince Alfred Hospital (RPAH). This embodied with a dedicated capacity to deliver approved and emerging advanced therapeutic medicinal products (ATMP). The GMP facility was opened in August 2012, qualified in June 2014, and has been servicing clinical trials through this facility since the first trial in October 2014. CMT has an outstanding record in clinical research and early adoption of novel technologies providing service to academic and industry partners for the development, manufacture, clinical evaluation and implementation of ATMPs.

SLHD has developed an integrated process and specified requirements for managing requests for the use of ATMPs within the health district. SLHD requires consultations with CMT's Head of Department and Research & Development Manager, RPAH Institutional Biosafety Committee and RPAH Pharmacy for all requests for the use of ATMPs within the district. This must be done in advance of the site being selected as a study site. For every clinical trial involving an ATMP, the Research & Development Manager conducts a feasibility study to ensure the facility has the capability and capacity to service the trial. With the growth in the number of clinical trials involving ATMPs, the number of trials serviced through CMT has been increasing exponentially. As such, the process for accessing CMT to provide services to a clinical trial was updated in 2020 to reflect the increase in demand for services.

Storage, formulation, processing, and dispensation of ATMPs for clinical trials is performed by the CMT production team, headed by a production manager. All production personnel are fully trained to operate according to the Australian code of GMP for human blood and blood components, human tissues, and human cellular therapy products. New personnel undergo an in-house training program which requires approximately 6 months to complete such that they are fully trained in all procedures performed in the facility. Personnel must attend each study specific Site Initiation Visit (SIV) for any new trials involving ATMPs within the district to ensure they have been trained to properly handle the investigational product (IP). In addition to the SIV, personnel are provided with an IP handling manual which includes all details for handling, storage, processing, and formulation of the IP. In addition, forms may also be provided by the sponsor unique to the individual trials, these must be maintained in accordance with the IP manual. Coordination and scheduling of patients to received ATMPs involves both clinical trials personnel from the department conducting the trial and CMT personnel to ensure available facility capacity and personnel on the scheduled date and time. Since qualification, CMT has serviced 22 clinical trials involving ATMPs with 11 still active, with many more in the pipeline.

BIOGRAPHY

Dr. Sharon Sagnella joined the Department of CMT at RPAH as the research and development manager in 2021. She joined CMT with 12 years of process development and commercialisation experience within CSIRO and industry where she contributed extensively to preclinical development, clinical trials, and the implementation of new therapies, combined with a successful academic research career. She has experience in engaging with investors and pharmaceutical company collaborators and was responsible for facilitating significant investment (financial amounts confidential) within industry. She transferred her industry experience to CMT, where she is responsible for overseeing the cell and gene therapy GMP facility at RPAH as well as liaising with clinical departments to enable clinical trials involving cell and gene therapies. She is also involved in facilitating cell therapy process development with academia and industry to translate research from bench to bedside and is a Chief Investigator on a $7 million MRFF grant awarded in 2022 for the development and commercialisation of tissue engineered cartilage. She is an Editorial Board member for Cytotherapy, an Associate Editor for Frontiers in Drug Delivery, and a member of the Royal Prince Alfred Hospital Institutional Biosafety Committee.

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SPEAKER PROFILE

DR. JANET MACPHERSON

Business Development Manager,
Enterprise Solutions Cell & Gene Therapy,
Cytiva

ABSTRACT

Closing up your process for cGMP compliance

Going into the clinic with your cell-based product is to establish safety in first in human use Phase I, and to look for early signs of efficacy. In order to do this in a manner that will allow streamlined progression to Phase II efficacy studies, it is worth evaluating early the scale up or scale out potential of the proposed manufacturing process. This presentation will provide general guidance on the benefits of selecting reagents and manufacturing systems that can help 'close up' your manufacturing process, and introduce cGMP ready reagents, single use consumables, qualification requirements and the benefits of automation. Some example manufacturing platforms will be discussed.

BIOGRAPHY

Dr Janet Macpherson joined Cytiva 4 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.

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SPEAKER PROFILE

DR. OLIVIA HIBBITT

Director, Speciality Service and Technology Evaluation Unit,
NSW Ministry of Health

DR. FILIMON HAILE

Manager - Highly Specialised Therapies,
Speciality Programs Commissioning & System Improvement Division
Department of Health

ABSTRACT

The implementation of cell therapies poses several challenges and opportunities for state governments.

In this talk, presenters will discuss the experiences and challenges in commissioning and implementing of cell and gene therapies in New South Wales and Victoria.
The presentation will focus on assessment, reimbursement, data collection and management, and long-term data evaluations patient follow-up, commercial interactions from a state government perspective.

BIOGRAPHY

Dr. Olivia Hibbitt started her career as a research scientist working in the field of gene therapy before switching careers to medical writing and eventually health policy and technology evaluation. Her current role is leading the NSW approach to health technology assessment and implementation and the management of highly specialised therapies. She has a DPhil. in Pharmacology from Oxford University and a Master of Public Health from the University of Sydney.

Dr. Filimon Haile manages the highly specialised therapies program at the Victorian Department of Health. His broader work focuses on health technology, highly specialized therapies, and clinical genetics. Within the highly specialised therapies space, Dr. Haile's work centres on the assessment, commissioning, implementation and evaluation of these therapies in the Victorian public healthcare system. Dr. Haile has a keen interest in the use of real-world data to inform healthcare decision-making. His expertise ensures that these therapies are accessible, cost-effective, and evidence-based for patients in need.

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SPEAKER PROFILE

ANNE MAREE ENGLUND

Head of Strategic Policy Implementation,
Medicines Australia

ABSTRACT

Industry perspective on health technology assessment for cell and gene therapies

Health technology assessment (HTA) plays a crucial role in informing decision-making about the value of medical treatments, including cell and gene therapies. While these therapies have the potential to cure genetic diseases and deliver long and healthy lives to patients, their high cost and complexity pose challenges for HTA. This means that there is a long time between registration with the TGA and reimbursement on the Pharmaceutical Benefits Scheme (PBS) by the National Blood Authority or as a highly specialised therapy (under the NHRA) if administered in public hospitals, during which time Australian patients cannot readily access these therapies. This talk will provide an industry perspective on the current state of HTA for cell and gene therapies and highlight how the ongoing independent HTA Policy and Methods Review is a unique opportunity to improve Australian patients' access to these therapies.

BIOGRAPHY

Anne-Maree is passionate about the potential of innovative technologies to improve health outcomes. An engineer by trade, she also has a Masters in Public Policy, and combines a thorough knowledge of product development with a strong understanding of the broader health and innovation policy landscape. She started her career at the medical device innovator Cochlear, has been the Operations Manager at health IT startup Humanetix, and also spent several years as Policy Manager at pharmaceutical company MSD. She is currently Head of Strategic Policy Implementation at Medicines Australia.

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SPEAKER PROFILE

DAVID KNEEN

Co-Founder, CEO and Head of Commercial,
Celleo

ABSTRACT

The role of engineers in driving commercial suitability of cell-based medicines

Today's approved CGTs are arguably pathfinders without serious commercial expectations, focused on demonstrating real-world efficacy and establishing new models for the provision of critical care. The outcomes have been encouraging - for haematological cancers at least, there is now considerable evidence of curative potential. We're far from a sustainable industry though – the commercial dynamics need to evolve significantly before CGTs sit comfortably in the standard clinician's toolbox as an available, affordable instrument of care.

Engineering has traditionally played an 'implementor' role in the translation of novel science to commercial therapeutics. For CGTs, the relationship becomes more intimate – the science and engineering are more intertwined than ever before. Yet today, engineering maturity lags the biology significantly - cell therapy production is a curious mix of cottage industry meets industry 4.0. While that's not unreasonable for a promising yet nascent field, commercial manufacturing remains a prominent challenge for the CGT industry – it's time for the engineers to step up.

Engineers can play two roles in the transition to commercial CGTs – building the industry framework in readiness for an influx of new therapeutics, and preparing therapeutics under development for successful transition. In this session, we'll explore these roles, and review the gap between perfect-world translation and our reality today.

BIOGRAPHY

A growth-focused global business executive, David has driven the industrialisation of cell, gene and tissue therapies for over 20 years. His career highlights include supporting the manufacturing establishment of many of today's commercial Cell & Gene Therapies, across North America, Central Europe and Asia-Pacific.

David previously managed Invetech's Cell Therapy automation business, leading a global team delivering commercial-scale manufacturing automation for the world's leading biotherapeutics multinationals, developing bespoke manufacturing systems for emerging novel biotherapeutics at pre-clinical and early-clinical phases, and producing platform technologies for pioneering technology start-ups across the Cell Therapy landscape.

Initially a deeply technical engineer and scientist, David brings multi-sector leadership experience, including a period as a management consultant across industries as diverse as health, finance and primary industries, and has worked for and within businesses from Fortune 500s through start-ups. He focuses on establishing robust strategic, financial, and operational foundations, from which to build powerful collaborations and drive meaningful change for the CGT industry.

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SPEAKER PROFILE

DR. NICK SIMPSON

Medical Adviser,
Technology Assessment and Access Division,
Commonwealth Department of Health and Aged Care

ABSTRACT

Health technology assessment and price negotiations for cell based medicines

Some prominent gene-modified cellular therapies have been classified as 'high cost, highly specialised therapies', a term defined in the National Health Reform Agreement (Addendum 2020-2025) which sets out the funding framework for such therapies. The Commonwealth Department of Health and Aged Care has a central role in the Health Technology Assessment (HTA) of high cost, highly specialised therapies. Health Technology Assessment provide policy-makers, funders, health professionals and health consumers with the necessary information to understand the benefits and comparative value of therapies. There are now several examples of gene-modified cellular therapies that have undergone an HTA process through the Department, leading to recommendations by the Medical Services Advisory Committee about public subsidy. This talk focuses on some key issues identified in those HTA processes and related negotiations.

BIOGRAPHY

Dr Nick Simpson has been a Medical Adviser in the Australian Government Department of Health and Aged Care's Technology Assessment and Access Division since 2018. Before joining the Division, he worked in the regulation of prescription medicines at the Therapeutic Goods Administration. He has qualifications in medicine, public health, and medical science.

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SPEAKER PROFILE

DR. KILIAN KELLY

Chief Operating Officer,
Cynata

ABSTRACT

Development of Class IV biological cell therapies

In accordance with the Australian Biologicals Regulatory Framework, which came into effect on 31 May 2011, Class IV biologicals are defined as "high risk" biologicals. Products falling into this category include some of the most cutting-edge stem cell and regenerative medicine products, including products derived from pluripotent stem cells, as well human cells or tissues that have been modified to artificially introduce a function that is not intrinsic to the cells or tissues. This classification scheme is unique to Australia. Of note, clinical trials using a Class IV biological must get approval through the TGA's Clinical Trial Approval (CTA) scheme, unless certain exemptions apply. This is a potentially significant impediment to clinical development of Class IV biologicals in Australia, as the CTA scheme requires substantially more preparation time and effort than the alternative Clinical Trial Notification (CTN) scheme, while the CTA review and approval timelines are lengthy, and the TGA review fee is also significantly higher. However, by understanding and navigating the specific exemptions that are available, it is possible to streamline and expedite development of Class IV biologicals, while retaining Australian centres as an integral part of a global development program.

BIOGRAPHY

Dr Kilian Kelly is Chief Operating Officer at Melbourne-based stem cell and regenerative medicine company, Cynata Therapeutics Limited. He has over 20 years' experience in biopharmaceutical research and development. Prior to joining Cynata in 2014, he held positions at a range of small and large companies, including Biota Pharmaceuticals, Mesoblast Limited, Kendle International, Amgen and AstraZeneca. He holds a Masters in Pharmacy from Robert Gordon University, Aberdeen and a PhD in Pharmaceutical Sciences from Strathclyde University, Glasgow. He is a registered pharmacist and a member of the Royal Pharmaceutical Society, the Australian Institute of Company Directors (AICD), and the International Society for Cell and Gene Therapy (ISCT). He also serves on the Industry Interface Committee of the Center for Commercialisation of Regenerative Medicine (CCRM) Australia.

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SPEAKER PROFILE

PROF. MINGHAO ZHENG

PCo-founder of Orthocell (ASX:OCC) and
Marine Biomedical Pty Ltd, Centre for Orthopaedic Research, Medical School,
The University of Western Australia

ABSTRACT

Market approval and implementation of cell therapies

Cell therapies for treatment of orthopaedic-related conditions have been well developed during the last 20 years. But there are only a few products that have been approved by regulatory agents. We were involved in the development of matrix-induced autologous chondrocyte implantation (MACI) which gained approval by FDA in 2016 as the first cell-scaffold combined product for cartilage repair. We subsequently developed another cell therapy product, autologous tendon cell injection for treatment of chronic degenerative tendinopathy. This talk covers the current state of cell therapy development using autologous and homologous approaches. The challenges of cell manufacture for market approval will be discussed. These include validation of multifactorial complex mechanisms of action, cell source, inherent cell characteristics, culture method, administration mode and the in vivo conditions to which the cells are exposed. We will also discuss development of bioassays used to measure biological activity, identity, purity and potency assay development for quality attributes in manufacturing. Design of clinical trials for assessment of efficacy of cell therapy will also be discussed. These include the implementation of step-wise induction of clinical studies from single arm studies to double blinded randomised control studies and real-world data (RWD).

BIOGRAPHY

Prof. Minghao Zheng has made significant contributions in the understanding of fundamental bone biology and translating research into industry and commercialisation.

After studying medicine at Shantou University and Sun Yat-sen University of Medical Sciences in China, Prof. Zheng came to The University of Western Australia in 1989, receiving a PhD in 1993 and Doctor of Medicine in 2000. He is fellow of the Royal College of Pathologists, UK and the Royal College of Pathologists of Australasia. He is a member of Faculty 1000 Prime and Associate Editor of Stem Cell Research and Therapy.

Prof. Zheng holds the position of Winthrop Professor of Orthopaedic Research at UWA.

Prof. Zheng's career has centred on bone and joint research and regenerative medicine. He pioneered the development of collagen scaffolds and cell therapy to treat cartilage, tendon and ligament injury, and is a global leader in regeneration of tendon and cartilage using a patient's own stem cells. He developed the Matrix-induced Autologous Chondrocyte Implantation (MACI) cartilage repair protocol and Autologous Tenocyte Therapy (ATT) for tendon repair. His work on giant cell tumour of bone is used by the World Health Organisation for the official classification of bone tumours. He has published over 250 papers and holds eight patented grants in Australia, USA, Japan, Singapore, Europe, and China.

In addition to academic and university leadership roles, Prof. Zheng is Co-Founder and Consultant Chief Scientific Officer of Orthocell Limited (ASX:OCC), a biomedical healthcare company.

SPEAKER PROFILE

DR. SILVIU ITESCU

Chief Executive Officer & Managing Director,
Mesoblast

ABSTRACT

Bringing MSC products for FDA approval

Mesoblast's lead product candidate, Ryoncil (remestemcel-L), has been optimised through manufacturing enhancements to have substantially greater potency than the first generation Prochymal product used in earlier studies. In an Expanded Access Protocol over more than ten years in children with steroid refractory acute graft versus host disease (SR-aGVHD), treatment with Ryoncil resulted in significantly greater survival outcomes than Prochymal. Mesoblast filed a Biologics License Application (BLA) in 2020 for Ryoncil which contained consistent treatment responses and survival outcomes across 309 children in three separate trials, including a Phase 3 trial that successfully met its primary endpoint. The data were reviewed by FDA's panel of the Oncologic Drugs Advisory Committee (ODAC) which voted in favor 9:1 that the available data support the efficacy of remestemcel-L in pediatric patients with SR-aGVHD. Despite the positive ODAC vote, the company received a complete response letter (CRL) in September 2020.

Mesoblast maintained an active dialogue with FDA since receiving the CRL, particularly in regard to addressing potency assay and chemistry, manufacturing and controls (CMC) items identified in the CRL. In January this year, Mesoblast resubmitted the BLA with substantial new information as required by FDA. The BLA resubmission contains new data on an optimized potency assay that was in place at the time of the Phase III trial, demonstrating a relationship between the product's activity *in vitro* and its effects on survival. Additional clinical and biomarker data include a propensity-matched study of children with high-risk disease and results of a 4-year survival study on 51 evaluable patients with SR-aGVHD who were enrolled in the Phase III trial.

In March, the FDA accepted the BLA resubmission as a complete response and has set a Prescription Drug User Fee Act (PDUFA) goal date of August 2, 2023.

BIOGRAPHY

Dr. Itescu has served on Mesoblast's Board of Directors since the Company's founding in 2004, was Executive Director from 2007, and became Chief Executive Officer and Managing Director in 2011. Prior to founding Mesoblast in 2004, he established an international reputation as a physician scientist in the fields of stem cell biology, autoimmune diseases, organ transplantation, and heart failure. Dr. Itescu has been a faculty member of Columbia University in New York, and the University of Melbourne and Monash University in Australia. In 2013, Dr. Itescu received the inaugural Key Innovator Award from the Vatican's Pontifical Council for Culture for his leadership in translational science and clinical medicine in relation to adult stem cell therapy. In 2011, he was named BioSpectrum Asia Person of the Year. He has consulted for various international pharmaceutical companies, has been an adviser to biotechnology and health care investor groups, and has served on the Board of Directors of several publicly listed life sciences companies.

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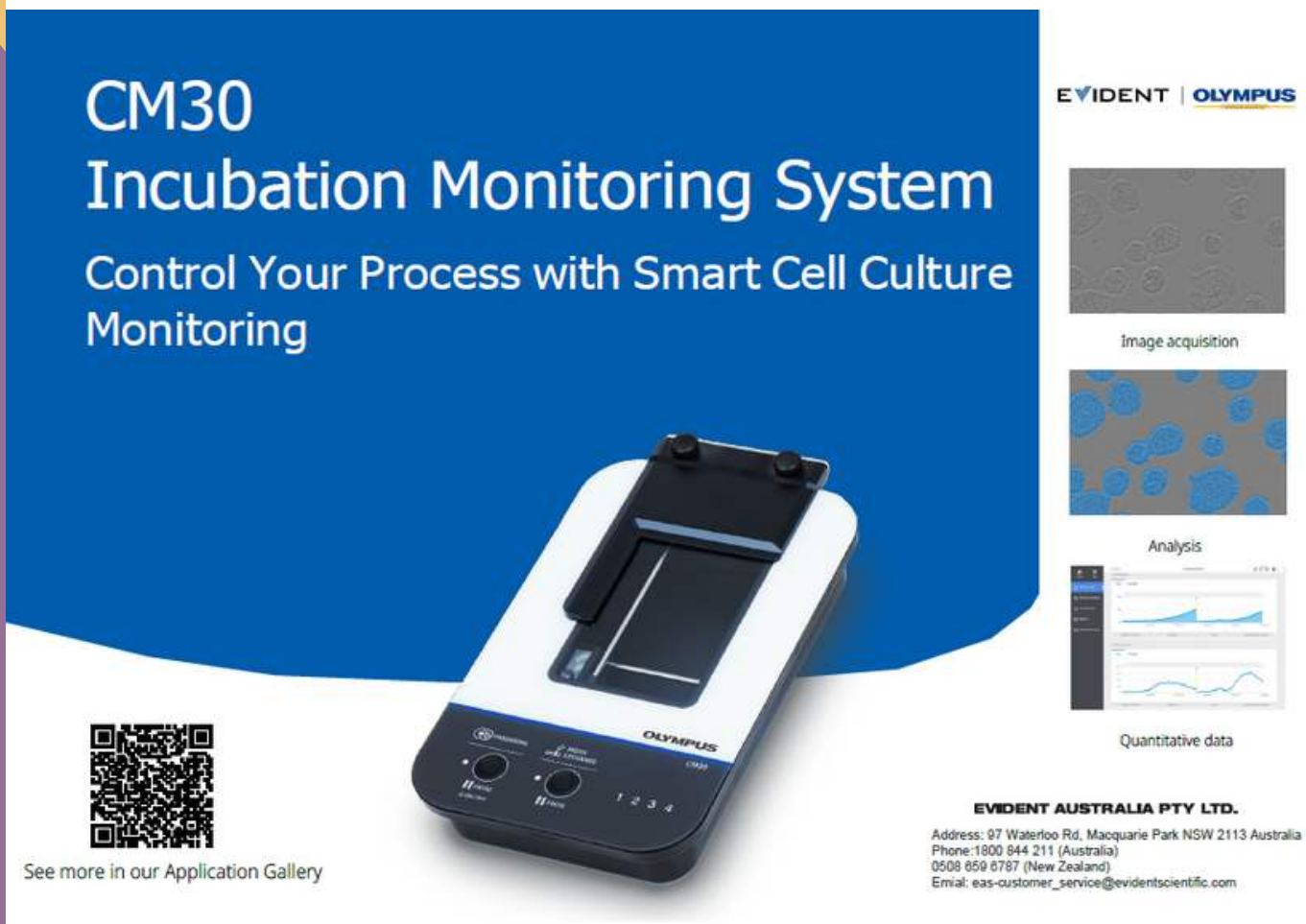
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