CELL & GENE THERAPY R&D CONGRESS: ASIA

17-18 APRIL 2024 | HOTEL FORT CANNING

Cell and gene therapies represent a transformative paradigm shift in our approach to disease treatment. These cutting-edge therapeutic modalities hold the promise of effectively addressing a spectrum of ailments for which treatment options were historically constrained. Notably, the Asia Pacific region has emerged as a leading proponent of this pioneering field, with numerous nations committing substantial resources to the research and development of these therapeutic interventions. This noticeable trend in Asia is accompanied by a surge of creative solutions entering the arena. The relentless growth of CGTs will not only revolutionize the approach to treating challenging diseases but also reshape the pharmaceutical landscape as we currently understand it.

The **2nd Cell & Gene Therapy Research & Development Congress Asia** aims to gather leading experts from the Cell & Gene Therapy field to share their latest scientific developments, clinical stage studies and triumphs at this conference.

2024 Speakers

- **Ivan Horak**, Founder & CEO, **Tikva Allocell**, Singapore
- **Greg Kunst**, Chief Executive Officer, **Aurion Biotech**, USA
- **Xianmin Zeng**, CEO & President, **RxCell Inc.**, Singapore
- **Minh Le**, Scientific Director & Cofounder & Assistant Professor and Graduate Program Director, **Carmine Therapeutics & National University of Singapore**, Singapore
- **Sandy Qlintang**, Director, **Stem Cell and Cancer Institute**, PT Kalbe, Indonesia
- **Jae Young Lee**, Director of R&D Therapeutics, **Toolgen**, South Korea
- **Cheng- Yi (Jerry) Kuo**, Vice General Manager, **UWELL Biopharma**, Taiwan
- **Dinender Singla**, Professor and Head, **University of Central Florida**, USA
- **Maxine Lam**, Senior Scientist I, **A*STAR**, Singapore
- **Sunil Gadekar**, Director Manufacturing Science and Technology Advanced Therapies, **Johnson & Johnson**, Singapore
- **Diego Laderach**, Professor and Head of Molecular and Functional Glyco-Oncology Lab, **Instituto de Química Biológica de la Facultad de Ciencias Exactas y Naturales de la Universidad de Buenos Aires,** Argentina
- **Yu Liu,** Head of R&D**, Wuhan Bio-Rad Biotechnology Co., Ltd.,** China
- **Jagadish Sankaran**, Scientist, **A*STAR**, Singapore
- **Christy Ma**, Chief Strategy Officer, SCG Cell Therapy, Singapore
- **Roumen Bogoev**, Head of Segment Marketing, **GenScript USA Inc.**, USA
- **Alex Hastie**, Vice President of Clinical and Scientific Affairs, **Bionano**

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0850 Welcome Remark from Global Engage

0900 **Keynote Presentation**
Minh Le, Scientific Director & Cofounder & Assistant Professor and Graduate Program Director, Carmine Therapeutics & National University of Singapore, Singapore

DELIVERY OF NUCLEIC ACID THERAPEUTICS USING EXTRACELLULAR VESICLES FROM RED BLOOD CELLS
Despite ongoing innovation and a small number of clinically successful applications, the delivery of nucleic acid-based therapeutics (NATs) remains challenging. Between the point of administration and the intended site of action, NATs have to contend with immune activation and clearance, degradation by nucleases, off-target accumulation, and various barriers posed by cellular membranes. We envision a delivery platform which can help NATs overcome these obstacles and realise their promise of specific and potent therapeutic effects. Nano-sized particles known as “red blood cell-derived extracellular vesicles” (“RBCEVs”) have proven to be an attractive candidate. Our research group has developed an inexpensive and scalable method to produce and purify RBCEVs from RBCs available in blood banks. Thanks to their cellular rather viral or synthetic origin, RBCEVs possess high biocompatibility and low immunogenicity. Our studies have demonstrated robust RBCEV-based delivery of RNAs, including small interfering RNAs and antisense oligonucleotides, to suppress leukemia as well as solid cancers and to treat cancer cachexia. Moreover, we have developed techniques to conjugate antibodies and peptides for enhanced specific targeting. In this talk, I will present the highlights of our works and discuss the unique potential of the RBCEVs as a new versatile drug delivery platform.

0930 **Keynote Presentation**
Christy Ma, Chief Strategy Officer, SCG Cell Therapy, Singapore

Topic: TBC
Abstract: TBC

1000 Alex Hastie, Vice President of Clinical and Scientific Affairs, Bionano

A NEW PARADIGM IN CELL AND GENE THERAPY - STRUCTURAL ASSESSMENT OF THE GENOME WITH OPTICAL GENOME MAPPING

- Safety concerns in cell and gene therapy
- Genetic assessment options
- Optical genome mapping (OGM) is a new tool for cytogenomic assessment.
- OGM in cell and gene therapy research for on- and off-target genome assessment.

1030 Morning Refreshments / Poster Presentations / One-to-One Meetings

1140 **Panel Discussion**
COMMERCIALIZATION AND INVESTMENT TRENDS IN ASIAN GENE THERAPY: OPPORTUNITIES AND RISKS
This panel discussion focuses on the ever-evolving dynamics of commercialization and investment in gene therapy within Asian markets. It explores the expanding opportunities and challenges that businesses and investors encounter as they navigate the promising field of gene therapy in Asia. Industry experts and leaders will provide valuable insights into the potential for growth and the associated risks, shedding light on the unique developments in this vital sector.

Moderator: Sunil Gadekar, MSAT Director, Advanced Therapies, Johnson & Johnson, Singapore
Panelist: Cheng- Yi (Jerry) Kuo, Vice General Manager, UWELL Biopharma, Taiwan
Panelist: Xianmin Zeng, CEO & President, RxXell Inc., Singapore

1210 Diego Laderach, Professor and Head of Molecular and Functional Glyco-Oncology Lab, Instituto de Química Biológica de la Facultad de Ciencias Exactas y Naturales de la Universidad de Buenos Aires, Argentina

NEW IMMUNOTHERAPY FOR PROSTATE CANCER

The design and evaluation of new immunotherapies for prostate cancer require a better understanding of the multiple molecular interactions between tumor cells and their associated microenvironment. Previously, we demonstrated that prostate carcinoma produces Galectin-1 as one of the key molecules to escape the immune attack. Therefore, it is essential to understand all the molecular processes in which this protein is involved. Most of the previous studies found in the literature have focused on the remodeling properties of the immune microenvironment by Galectin-1 secreted by the tumor, through its interactions with glyco-receptors at the cell membrane and the extracellular matrix. In this conference, I propose to discuss recent results of our research group that demonstrate a novel role of endogenous Galectin-1 in T lymphocytes in the control of anti-prostate cancer immunity. Indeed, using a preclinical murine model of prostate cancer, our results show that Galectin-1 of lymphocytes plays a fundamental role in the regulation of their proliferation rates and cytotoxic function. This functional regulation of lymphocyte Galectin-1 occurs under conditions of high concentration of extracellular Galectin-1, which is derived mainly from tumor cells. Under such conditions, the absence of Galectin-1 in T lymphocytes enhances anti-tumor immune responses. Our results demonstrate that endogenous Galectin-1 in CD4 + T lymphocytes, but mainly in CD8 + T cells, acts as a negative regulator of anti-tumor immunity. In conclusion, prostate tumors require T lymphocytes to express Galectin-1 to evade immune responses. These results lay the foundation for an original immunotherapy strategy for prostate cancer.

1235 **Solution Provider Presentation**

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1305 Lunch / Poster Presentations / One-to-One Meetings

1405 Maxine Lam, Senior Scientist I, A*STAR, Singapore

AN ADVANCED IN VITRO MODEL FOR GLIOBLASTOMA AND THE BLOOD-BRAIN BARRIER FOR STUDYING TUMOUR-VASCULATURE INTERACTION, DRUG RESISTANCE AND CAR-T CELL THERAPY EFFICACY

Glioblastoma (GBM) has a dismal prognosis and limited chemotherapeutic options, despite decades of research. Major obstacles to targeted therapy include GBM intra- and inter-tumour heterogeneity, as well as drug delivery across the blood-brain barrier (BBB). We developed a microphysiological in vitro model that recapitulates important clinical features of GBM such as a dense tumor core with a diffused invasive front and a perfusable BBB with a physiological permeability and morphology that is altered by the tumor. The model enabled studies on how tumour heterogeneity and the BBB affect temozolomide (TMZ) sensitivity, and proteomic analysis of microtumors with immortalized and patient derived GBM progenitor cells suggests that co-culture with a BBB increases tumour aggressiveness and TMZ resistance. The model can also be used to validate novel CAR-T cell targeting and cytotoxicity, and is compatible with downstream analysis of the tumour microevironment and CAR-T states using flow cytometry and single cell -omics.

1430 Jae Young Lee, Director of R&D Therapeutics, Toolgen, South Korea

THERAPEUTIC GENOME EDITING
- In vivo therapeutic genome editing
- Ex vivo therapeutic genome editing
- Regulation of therapeutic genome editing

1455 Roumen Bogoev, Head of Segment Marketing, GenScript USA Inc., USA

Transforming disease to therapy – GenScript's One-Stop Solution for Cell and Gene Therapy Development
Gene and cell therapy are promising new fields of medicine that aim to treat or prevent diseases by manipulating genes and cells. Gene therapy involves using genetic material to treat or prevent diseases by replacing, inactivating, or introducing genes into cells. Cell therapy, on the other hand, involves the transplantation of cells into a patient's body to replace damaged or diseased cells. Both gene and cell therapies have shown great potential in treating a wide range of diseases, including cancer, genetic disorders, and autoimmune diseases. However, many challenges still remain to the development and manufacturing of such therapies. GenScript offers an end-to-end workflow composed of advanced services and products to facilitate the development and manufacturing of such therapies. In this presentation, we will review our solutions for Target Discovery, Lead Generation and Optimization, and Pre-clinical and Clinical Development of gene and cell therapies.

1525 Afternoon Refreshments / Poster Presentations / One-to-One Meetings

1615 **Panel Discussion**
REVOLUTIONIZING HEALTHCARE: THE ART OF AFFORDABLE EXCELLENCE IN CELL & GENE THERAPIES

Efforts to attain both affordability and excellence in the field of cell and gene therapies represent a fundamental objective in healthcare. The core mission is to make state-of-the-art therapies available to a wider population while upholding rigorous standards of quality and therapeutic efficacy. In this panel discussion, experts from the field will share insight on developing therapies that are both exceptional and accessible. Through pioneering approaches, the panel aims to redefine the future of healthcare, ensuring that groundbreaking treatments reach those in need and reshape the healthcare landscape for the better.

Moderator: Sunil Gadekar, MSAT Director, Advanced Therapies, Johnson & Johnson, Singapore
Panelist: Greg Kunst, Chief Executive Officer, Aurion Biotech, USA
Panelist: Dinender K. Singla, Professor & Head of Metabolic & Cardiovascular Sciences, University of Central Florida

1645 Dinender K. Singla, Professor & Head of Metabolic & Cardiovascular Sciences, University of Central Florida, USA

EXOSOMES AMELIORATE DOXORUBICIN INDUCED CARDIAC AND MUSCLE TOXICITY
Doxorubicin (Dox) has long been hailed as an effective chemotherapeutic drug, offering hope to countless cancer patients. However, its potential has been limited by the occurrence of serious side effects, particularly cardiac and skeletal muscle toxicity. Dox-induced muscle toxicity (DIMT) occurs due to enhanced oxidative stress, inflammation, and excessive apoptosis and necrosis. This is not established whether DOX induces inflammatory mechanisms that induces cardiac muscle cellular cross talk mediated toxicity. Moreover, the role of embryonic stem (ES) cells derived exosomes in the inhibition of DOX induced muscle toxicity is not examined. We will provide evidence that established cardiac and soleus muscle toxicity involves inflammation, atrophy, and adverse structural remodeling. These inflammatory mechanisms and their impact on structural changes with decreased physiological function were examined using histology, immunohistochemistry, real-time polymerase chain reaction (RT-PCR).

Additionally, Cytokine array (90-cytokines) was performed for all the groups to evaluate in depth involvement of inflammatory factors. Our data shows treatment with Dox significantly reduced cardiac and muscle strength which was associated with increased expression of pro-inflammatory cytokines (TNF-α and IL-6), increased inflammatory M1 macrophages and reduced anti-inflammatory M2 macrophage markers compared with controls. Further, an increase in inflammatory cytokine profiles, muscular atrophy, fibrosis, structural alterations were observed following Dox treatment. Exosomes significantly ameliorates these altered inflammatory cellular mechanisms, cell death, enhances anti-inflammatory cell signaling pathways. We also report that exosomes contain a unique set of anti-inflammatory cytokines which attenuate inflammation, fibrosis with improved function which could have a potential in the clinic.

1710 Jagadish Sankaran, Scientist, Agency for Science, Technology and Research (ASTAR), Singapore

IDENTIFICATION OF MORPHOGENOMIC PHENOTYPES USING SPATIAL OMICS IN CHRONIC MYELOMONOCYTIC LEUKEMIA

Chronic myelomonocytic leukemia is a myeloid neoplasm characterized by aberrant dysmorphology of monocytic nuclei (myelodysplasia) and increase in the proportion of monocytes in white blood cell count (myeloproliferation). Nuclear dysmorphology coupled with aberrations in gene expression are hallmarks of CMML. However, these biomarkers have not been jointly analyzed on a large scale. Here, we have developed a spatial omics assay of 492 genes in ~25,000 cells in a 16 mm2 region of interest in 34 hours for simultaneous investigation of nuclear dysmorphology and aberrant gene expression in bone marrow mononuclear cells (BMMC) obtained from blood malignancies. Next, we applied the assay to BMMC samples from 12 patients with CMML and performed bimodal nuclear shape and gene expression analysis to identify novel morphogenomic phenotypes. Combining morphological analysis with gene expression could improve CMML patient stratification and reveal underlying biology of clinically relevant malignant cell morphologies.

1735 Closing Remarks and Drinks Reception

Poster Presentations

Poster presentation sessions will take place in breaks and alongside the other breakout sessions of the conference. Your presentation will be displayed in a dedicated area, with the other accepted posters from industry and academic presenters. We also issue a poster eBook to all attendees with your full abstract in and can share your poster as a PDF after the meeting if you desire (optional). Whether looking for funding, employment opportunities or simply wanting to share your work with a like-minded and focused group, these are an excellent way to join the heart of this congress. In order to present a poster at the forum you need to be registered as a delegate. Please note that there is limited space available and poster space is assigned on a first come first served basis (subject to checks and successful registration)

Submission instructions

We will require the form to be submitted by **22nd March 2024**. This is the formal deadline however space is another limiting factor so early application is recommended. Therefore please contact us with any questions you have as soon as possible.

CONGRESS SCHEDULE 18 APRIL 2024

0850 Welcome Remark from Global Engage

0900 **Keynote Presentation**
Greg Kunst, Chief Executive Officer, Aurion Biotech, USA

THE JOURNEY FROM AN ACADEMIC CELL THERAPY APPROACH TO HIGHLY SCALED COMMERCIAL OPPORTUNITY
To explore the challenges and opportunities in moving an academic cell therapy program into a full-scale program ready to meet global demands and needs. Expectations when bringing in a university developed cell therapy into an active development program. Challenges associated with pre-clinical, clinical, and CMC needed to progress development pathway. Needed team members to advance program. Implications to tech transfer and how to engage with university developers once transfer is complete.

0930 **Keynote Presentation**
Ivan Horak, Founder & CEO, Tikva Allocell, Singapore

CHALLENGES AND OPPORTUNITIES OF CELL THERAPY IN ASIA
Abstract: TBC

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1030 Morning Refreshments / Poster Presentations / One-to-One Meetings

1140 **Panel Discussion**
ASIA'S ROLE IN SHAPING THE FUTURE OF CELL THERAPY: INNOVATION, COLLABORATION, AND MARKET TRENDS
This panel discussion will explore the significant and ever-evolving role of Asia in shaping the future of cell therapy. With an emphasis on innovation, collaboration, and market trends, it will bring together experts and thought leaders to delve into the unique contributions and challenges facing the region. As cell therapy rapidly advances, Asia has emerged as a dynamic force in this field, playing a pivotal role in global developments.
Panelist: Cheng- Yi (Jerry) Kuo, Vice General Manager, UWELL Biopharma, Taiwan
Panelist: Ivan Horak, Founder & CEO, Tikva Allocell, Singapore

1210 Xianmin Zeng, CEO & President, RxCell Inc., Singapore

Developing of iPSC-derived retinal progenitor cell therapy for retinal degenerative diseases
RxCell is a biotechnology company focused on therapeutic applications of induced pluripotent stem cells (iPSC). We have manufactured current Good Manufacture Practice (cGMP) iPSC master cell banks (MCB) for clinical use. Currently, we are in the process of manufacturing retinal progenitor cells from one of our iPSC MCB for an IND (investigational new drug) application with the US FDA for treating retinal degenerative diseases. I will discuss our IND-enabling activities for a Phase-I dose-escalation study. In addition, we have generated hypoimmunogenic (universal) iPSC lines as a safe universal donor cell source for allogeneic therapies for next generation cell therapy products, and I will discuss our recent advance in this novel technology.

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1305 Lunch / Poster Presentations / One-to-One Meetings

1405 Sandy Qlintang, Director, Stem Cell and Cancer Institute, PT Kalbe, Indonesia

MESENCHYMAL STEM CELLS AND THEIR SECRETOME: THE RATIONALE FOR PULMONARY REGENERATION

Acute and chronic pulmonary diseases, such as chronic obstructive pulmonary disease, pulmonary fibrosis, and pulmonary hypertension, are considered significant global health issues. Cellular therapies utilizing Mesenchymal Stem Cells (MSCs) present a novel therapeutic approach for both chronic and acute lung conditions, leveraging their anti-inflammatory, immunomodulatory, regenerative, pro-angiogenic, and anti-fibrotic properties. These therapeutic effects are associated with the MSC-secretome, comprising freely soluble proteins and extracellular vesicles (EVs).

Recent findings regarding the efficacy and safety of MSC-derived products in pulmonary diseases underscore the biologically active substances within the MSC-secretome and the mechanisms involved in tissue regeneration. MSC and MSC-secretome hold potential as innovative and transformative approaches in treating pulmonary diseases, offering hope for improved outcomes for individuals. Further clinical trials are essential to validate the therapeutic potential of MSC-secretome and MSC in the realm of pulmonary diseases.

1430 Yu Liu, Head of R&D, Wuhan Bio-Rad Biotechnology Co., Ltd., China

CD99 SPECIFIC CAR-T CELLS FOR THE TREATMENT OF SARCOMA

CAR-T cell therapy has shown dramatic clinical success in relapsed or refractory B-ALL and other hematological malignancies. However, the loss of tumor specific antigens, poor immune cells infiltration, and highly immunosuppressed tumor microenvironment are challenges in treating solid tumors with CAR-T therapy. Our previous data indicated that CD99 is a promising antigen to target T-ALL and AML, and an isolated low-affinity CD99 (12E7) antibody could specifically recognizes leukemia cells over normal blood cells. Moreover, the anti-CD99 CAR-T cells showed robust cytotoxicity specifically against CD99+ T-ALL cell lines and primary tumor cells in vitro and in vivo. Interestingly, CD99 is highly expressed in sarcoma samples, especially in Ewing's sarcoma (ES). Our new work demonstrated that anti-CD99 CAR-T had cytotoxicity specifically against CD99+ sarcoma cell lines and primary tumor cells in vitro and significantly prolonged CDXs or PDXs models survival in vivo. What's more, two sarcoma patients who infused with anti-CD99 CAR-T cells have showed better response rate, and have no serious adverse reactions.

1455 Cheng- Yi (Jerry) Kuo, Vice General Manager, UWELL Biopharma, Taiwan

THE DEVELOPMENT STORY OF UWC19 (WELGENALEUCEL) FOR TREATING B CELL MALIGNANCIES: FROM BENCH TO CLINICAL BED

Genetically modifying autologous T cells to express an anti-CD19 chimeric antigen receptor (CAR) has emerged as a promising treatment for CD19+ B cell malignancies with impressive response rates. UWELL Biopharma developed CAR-T treatment, UWC19 (Welgenaleucel), a cellular therapy consisting of autologous T cells transduced with an anti-CD19 chimeric antigen receptor for the patients with relapsed or refractory CD19 positive Acute Lymphoblastic Leukemia (ALL) and Diffused Large B Cell Lymphoma (DLBCL). UWC19 has been evaluated for it pharmacological properties and toxicological effects. The results showed that UWC19 effectively eliminated CD19+ cancer cells in xenograft animals and that UWC19 had limited off-target toxicity and tumorigenic potential. Taken favorable and acceptable, pre-clinical results, UWC19 had been investigated for its safety and efficacy in patients with B cell malignancies through clincial trials.

1520 Closing Remarks and End of Conference