GRADUATE CERTIFICATE IN HEALTH PRODUCTS REGULATION

GMS5005: Regulation of Advanced Therapies

15 April 2024 – 19 April 2024

Venue: Whitespace at Academia (SGH)

WORKSHOP PROGRAMME

Learning outcomes

- Define and categorize the scope of different ATMPs across different regulatory agencies and distinguish them from conventional pharmaceutical products.
- Describe the critical components in the production and administration of ATMPs including manufacturing, nonclinical/clinical development
- Explain the basis of the premarketing quality and clinical regulatory requirements across ATMP product lifecycle.
- Distinguish between the established ATMP regulatory frameworks across the globe and the various initiatives to promote regulatory harmonization.

Graduate Certificate in Pharmaceutical Regulation

GMS5005: Regulation of Advanced Therapies

15 – 19 April 2024

Day 1 – 15 April, Mon

	Topic	Speaker/ Organisation
8.30am	**Welcome Graduate Certificate Students**	**Prof John Lim** Executive Director Centre of Regulatory Excellence (CoRE) Duke-NUS Medical School
8.50am	**Workshop Briefing**	**Dr Uttara Soumyanarayanan** Senior Education Associate, CoRE
Session 1: Introduction to Advanced Therapy Medicinal Products (ATMPs)		
9.00am	**Overview of ATMPs** • Definition and scope of ATMPs • Examples of approved products • Gene therapy and CAR-T cell therapy	**Dr Kellathur Srinivasan** Regional Regulatory Policy Lead APAC Roche, Singapore
Session 2: Regulatory Frameworks for Advanced Therapy Medicinal Products (ATMPs)		
9.45am	**US Regulatory Frameworks of Regenerative Medicine Therapies** • US legal framework • Expedited pathway for RMTs	**Ms Judith Arcidiacono** International Regulatory Expert Center for Biologics Evaluation and Research, Office of Therapeutic Products, US FDA
10.45am	**Refreshment Break**	
11.00am	**TGA Regulatory Framework for ATMPs** • Biologics framework for ATMPs • MA Pathways for ATMPs	**Dr Natasha Brockwell** Director, Indo-Pacific Regulatory strengthening Program, Therapeutic Goods Administration, Australia
12.00pm	**Lunch**	
1.00pm	**Overview of HSA's CTGTP Regulatory Framework** • Scope of regulation • Class 1 and Class 2 products • Regulatory Controls	**Ms Christine Ho** Deputy Director, Advanced Therapy Products Branch Health Sciences Authority (HSA), Singapore **Dr Dorothy TOH Su Lin** Assistant Group Director Medicinal Products Pre-market Cluster HSA, Singapore
1.45pm	**PMDA Regulatory Framework for Regenerative Medicine** • PMDA legal framework • Considerations for expedited pathways • Examples of approved products	**Mr Yusuke Nozaki** Reviewer Office of Cellular and Tissue-based Products Pharmaceuticals and Medical Devices Agency (PMDA), Japan
2.40pm	**Refreshment Break**	
3.00pm	**EU Regulatory Framework for ATMPs** • EU Legal framework • ATMP marketing authorisation procedures • Post-authorisation requirements	**Dr Patrick Celis** Lead Scientific Officer - Committee for Advanced Therapies (CAT) at European Medicines Agency
4.00pm	**Practicum I:** • Classification and Categorisation of ATMPs	**Dr Kellathur Srinivasan** Roche
5.30pm	**End of Day 1**	

Day 2 – 16 April, Tue

	Topic	Speaker/ Organisation
8.30am	**Individual and Group assessment I**	
Session 3: CMC Considerations in ATMP Manufacturing		
9.30am	**Management of Raw Materials for ATMPs** • Starting materials vs raw materials: comparison across RAs • Risk-based approach • Role of pharmacopoeial standards in regulatory requirements	**Dr Fouad Atouf** Senior Vice President Global Biologics The United States Pharmacopeial Convention (USP)
10.40am	**Refreshment Break**	
11.00am	**Cell and Gene Therapy Manufacturing** • Overview of manufacturing • Ensuring scalability • Challenges in ATMP manufacturing	**Dr Lucas Chan** Scientific Founder & CSO CellVec Pte Ltd
12.15pm	**Lunch**	
1.15pm	**Manufacturing of CAR T-cell therapies** • Overview of CART • Manufacturing facility and process • Singapore's cell therapy ecosystem	**Dr Sudipto Bari** Head & Assistant Director, Translational Services & Regulatory Management, Advanced Cell Therapy and Research Institute, Singapore (ACTRIS)
2.30pm	**CTGTP Manufacturing Facility and GMP Inspections** • Key GMP Audit Areas • Examples of scenarios with non-compliance	**Mr Junaidi Abu** Regulatory Consultant (GMP) Audit & Licensing Division, HPRG Health Sciences Authority (HSA), Singapore
3.30 pm	**Refreshment Break**	
4.00pm	**Evaluation of CMC Dossier** • Quality attributes and requirements • Stability requirements • Challenges and considerations	**Dr Lee Lee Ong** Regulatory Consultant Advanced Therapy Products Branch Health Products Regulation Group Health Sciences Authority (HSA), Singapore
5.00pm	**End of Day 2**	

Day 3 – 17 April, Wed

	Topic	Speaker/ Organisation
8.30am	**Individual and Group assessment II**	
9.30am	**Practicum II** • Case studies: Raw Materials • Handling out of specifications (OOS) products	**Expert Faculty:** Dr Fouad Atouf, USP Dr Ong Lee Lee, HSA
10.30am	**Refreshment Break**	
11.00am	**Practicum II continued**	
12.15pm	**Lunch**	
Session 4: Nonclinical and Clinical Development and Evaluation of ATMPs		
1.15pm	**Nonclinical Evaluation of ATMPs** • Characteristics of ATMPs • Challenges of nonclinical assessment for ATMPs • Pharmacology and toxicology assessment • Additional data assessment	**Dr Xiaofeng WU** Regulatory Consultant Innovation Office and Clinical Trials Branch Health Sciences Authority (HSA), Singapore
2.15pm	**Case Discussion: Non-Clinical Assessment**	**Expert Faculty:** Ms Judith Arcidiacono
3.30pm	**Clinical Development of ATMPs** • Engagement with US FDA during planning • Design of early phase trials • Execution of trials	**Dr Steve Winitsky** Vice President, Technical PAREXEL Consulting, PAREXEL International, Arizona, US
4.00pm	**Refreshment Break**	
4.30pm	**Review of Clinical Trial Application** • Regulatory requirements for nonclinical and clinical trials • Components of IND submission • Challenges specific to RMTs	**Ms Judith Arcidiacono** US FDA
5.15pm	**End of Day 3**	

Day 4 – 18 April, Thurs

	Topic	Speaker/ Organisation
8.30am	**Individual and Group Assessment III**	
9.30am	**Practicum III: Clinical Development and Evaluation** • Clinical development studies • Benefit risk evaluation	**Expert Faculty:** Ms Judith Arcidiacono US FDA
10.40am	**Refreshment Break**	
11.00am	**Practicum III continued**	
12.00pm	**Lunch**	
1:00pm	**Practical Aspects of CAR-T cell therapies** • Concerns & challenges: monitoring, access, recruiting pediatric populations • Barriers and enablers impacting uptake	**Dr Bernice Oh** Consultant, Division of Paediatric Haematology and Oncology, National University Hospital
1.45pm	**Challenges with clinical efficacy assessments** • Benefit-risk assessment on limited database • Post-authorisation measures	**Dr Attila Sebe** Scientist, Clinical Assessor for ATMPs, Paul-Ehrlich-Institut (PEI), Federal Institute for Vaccines and Biomedicines, Germany
Session 5: Post-market Activities of ATMPs		
2:30pm	**Supply chain and distribution of ATMPs** • Maintaining cold chain supply • Quality deviations • Case examples	**Mr Paul TK Goh** National Business Development Manager World Courier **Mr Calvin Tan** Customer Success Project Coordinator World Courier
3.30pm	**Refreshment Break**	
3.45pm	**Pharmacovigilance and Risk Management of ATMPs** • Safety and efficacy concerns with ATMPs • Routine and additional PV activities • Long-term follow up studies	**Ms Phua Chwee Ping** Regional Head of Patient Safety, Asia-Pacific, Novartis
4:45pm	**Pre-panel polling session**	**CoRE Education Team**
5.15pm	**End of Day 4**	

Day 5 – 19 April, Fri

	Topic	Speaker/ Organisation
Session 6: Advanced Therapies Landscape: Challenges and Opportunities		
9:00am	**End of the Module Assessment**	**CoRE Education Team**
10:00am	**EOM Review and Clarifications**	**CoRE Education Team**
10.45am	**Refreshment Break**	
11.00am	**Patient Case Study: Treatment with CAR-T cells** Clinical utility of CAR-T cell therapies and challenges with treatment	**Dr Francesca Lorraine Lim** Senior Consultant Hematologist Singapore General Hospital (SGH) Chief Medical Officer, Advanced Cell Therapy and Research Institute, Singapore (ACTRIS)
12.00pm	**Lunch**	
1.00pm	**Pricing, Access and Reimbursement of ATMPs: Challenges and Barriers**	**Ms Liang Lin** Deputy Director/Senior Lead Specialist (Value-based Pricing and Gene Therapy Evaluation) Agency for Care Effectiveness (ACE), Ministry of Health (MOH), Singapore
1.45pm	**Regulatory Harmonization and Convergence Initiatives**	**Dr Kellathur Srinivasan** Roche
2:30pm	**Peer learning activity**	**CoRE Education Team**
3.15pm	**Refreshment Break**	
3.30pm	**Panel Session** **"Preparing the healthcare system for Advanced Therapies"**	**Moderator:** Dr Francesca Lorraine Lim **Panelists:** Ms Judith Arcidiacono Dr Anuradha Rajapakse (Sr Director, Market Access & Reimbursement, Gilead) Mr Samuel Soussi (Regional Director of Operations, World Courier)
4.30pm	**Graduate Certificate Workshop Conclusion**	**A/Prof Silke Vogel** Deputy Director, CoRE Senior Associate Dean Graduate Studies Duke-NUS Medical School
5.00pm	**End of Workshop**	