Centre of Regulatory Excellence
@ Duke-NUS Medical School

GRADUATE CERTIFICATE IN PHARMACEUTICAL REGULATION

GMS5103: Regulation of Cell, Tissue and Gene Therapies

18 January 2021 – 22 January 2021

Venue: Zoom

Day 1 **Mon, 18 January**	
Day 2 **Tues, 19 January**	
Day 3 **Wed, 20 January**	
Day 4 **Thurs, 21 January**	
Day 5 **Thurs, 22 January**	

Online Preparatory Materials

Topic 1 – Fundamentals of ATMPs

Topic 2 – Overview of Regulatory Frameworks

Topic 3 – Regulatory requirements of ATMPs

Topic 4 – PV of ATMPs

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

GMS5103: Regulation of Cell, Tissue and Gene Therapies

18 – 22 January 2021

Day 1 – 18 January, Mon

	Topic	Speaker/ Organisation
8.15am	**Zoom Briefing**	**Mr Osman Bin Mohamad** Senior Associate Centre of Regulatory Excellence (CoRE) Duke-NUS Medical School
Session 1: Introduction to Advance Therapy Medicinal Products (ATMPs)		
8.30am	**Welcome Graduate Certificate Students**	**Prof John Lim** Executive Director Centre of Regulatory Excellence (CoRE) Duke-NUS Medical School
8.40am	**Workshop Briefing**	**Dr Uttara Soumyanarayanan** Associate II CoRE, Duke-NUS Medical School
8.50am	**Overview of ATMPs** • Definition and scope of ATMPs • Examples of approved products • Gene therapy and CAR-T cell therapy	**Dr Patrick Celis** Scientific Administrator (CAT Secretariat) European Medicines Agency (EMA)
Session 2: Regulatory Frameworks for Advance Therapy Medicinal Products (ATMPs)		
9.30am	**US Regulatory Frameworks** • Regenerative Medicines • US legal framework • Expedited pathway for RMTs	**Ms Judith Arcidiacono** International Regulatory Expert and Standards Development Liaison Office of Cellular Tissue and Gene Therapy U.S. Food and Drug Administration (US FDA)
10.30am	**Refreshment Break**	
11.00am	**TGA Regulatory Framework for ATMPs** • Biologics framework for ATMPs • MA Pathways for ATMPs	**Dr Tony Manderson** Principal Advisor Cell & Tissue Therapies Unit Therapeutic Goods Administration (TGA)
11.45am	**Progress in ASEAN Frameworks for ATMPs** • Thai FDA • NPRA Malaysia	**Mr Wittawat Viriyabancha** Pre-marketing Control Division Bureau of Drug Control, Thailand FDA **Dr Azizah Abd Ghani** Head of Biologic Section NPRA, Malaysia
12.30pm	**Lunch**	
2.00pm	**PMDA Regulatory Framework for Regenerative Medicine** • PMDA legal framework • Considerations for expedited pathways • Examples of approved products	**Dr Yoshiaki Maruyama** Review Director Office of Cellular and Tissue-based Pharmaceuticals and Medical Devices Agency (PMDA)
2.30pm	**Refreshment Break**	
2.55pm	**Photo-taking with Faculty and Participants**	**CoRE Team**
3.00pm	**EU Regulatory Framework for ATMPs** • EU Legal framework • ATMP marketing authorisation procedures • Post-authorisation requirements	**Dr Patrick Celis** EMA

4.00pm	**Practicum I:** • Categorisation of ATMPs	**Dr Patrick Celis** EMA
5.30pm	**End**	

Day 2 – 19 January, 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 • Case Example	**Dr Fouad Atouf** Vice President Science—Global Biologics The United States Pharmacopeial Convention (USP)
10.30am	**Refreshment Break**	
11.00am	**Cell and Gene Therapy Manufacturing** • Overview of manufacturing • Ensuring scalability • Challenges in ATMP manufacturing	**Dr Lucas Chan** Co-founder CSO CellVec
12.00pm	**Lunch**	
1.00pm	**Commercial Manufacturing of ATMPs** • Manufacturing process development • Regulatory requirements: product characterisation of DS/DP, stability • Technology transfer and process validation	**Mr Xiangliang Lin** Founder and President Esco Healthcare
2.00pm	**ATMP Manufacturing Facility and GMP Inspections** • GMP compliance • Examples of scenarios with non-compliance	**Dr. Elaine Tang** Senior Regulatory Specialist (GMP) Audit & Licensing Division, HPRG Health Sciences Authority
2.45pm	**Introduction to HSA Cell Therapy Facilities**	**Dr Marieta Chan** Laboratory Director & Consultant Scientist HSA
3.15pm	**Refreshment Break**	
3.45pm	**Evaluation of CMC Dossier** • Quality attributes and requirements	**Dr Lee Lee Ong** Regulatory Consultant Advanced Therapy Products Branch Health Products Regulation Group HSA
5.30pm	**End**	

Day 3 – 20 January, Wed

	Topic	Speaker/ Organisation
8.30am	**Individual and Group assessment II**	
9.30am	**Practicum II** • Case studies: Raw Materials • Evaluation of CMC dossier	**Expert Faculty:** Dr Fouad Atouf Dr Ong Lee Lee, HSA
10.30am	**Refreshment Break**	
11.00am	**Practicum II continued**	
12.00pm	**Lunch**	

1.00pm	**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 HSA
2.00pm	**Case Discussion: Non Clinical Assessment**	**Expert Faculty:** Dr Xiaofeng WU
Session 4: Clinical Development of ATMPs		
2.45pm	**Clinical Development of ATMPs** • Engagement with US FDA during planning • Design of early phase trials • Execution of trials	**Dr Steve Winitsky** Vice President - Technical, Paraxel Consulting
3.15pm	**Refreshment Break**	
3.45pm	**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
5.00pm	**End**	

Day 4 – 21 January, Thurs

	Topic	Speaker/ Organisation
8.30am	**Individual and Group Assessment III**	
9.30am	**Practicum III** • Clinical development studies • Benefit risk evaluation	**Expert Faculty:** Dr Kellathur Srinivasan, HSA
10.30am	**Refreshment Break**	
11.00am	**Practicum III continued**	
12.00pm	**Lunch**	
1.00pm	**Challenges with clinical efficacy assessments** • Benefit-risk assessment on limited database • Post-authorisation measures	**Dr Martina Schüssler-Lenz** Chair, EMA Committee for Advanced Therapies, Paul-Ehrlich-Institut, Federal Institute for Vaccines and Biomedicines
2.00pm	**Case Study: Early phase development of T cell immunotherapy** • Preclinical study results • Case report: T-cell immunotherapy treatment protocol, safety and efficacy results	**Dr Anthony Tan** Senior Research Fellow Duke-NUS Medical School
3.00pm	**Refreshment Break**	
Session 5: Pharmacovigilance of ATMPs		
3.30pm	**Pharmacovigilance and Risk Management of ATMPs**	**Ms Phua Chwee Ping**, Head of Patient Safety, Regional PVO Manager, AMAC at Novartis
4.30pm	**Pre-panel Polling Activity**	CoRE Staff
5.00pm	**End**	

Day 5 – 22 January, Fri

	Topic	Speaker/ Organisation
Session 6: Challenges and Opportunities in ATMPs		
8.30am	**Challenges in the Development and Translation of ATMPs**	**Ms Katherine Tsokas** Vice President Regulatory Risk Management & Drug Safety

		Janssen
9.10am	**Challenges with Diversified Regulatory Guidelines and Requirements**	**Ms Chin Koerner** Executive Director US Regulatory and Development Policy Novartis
9.50am	**Challenges in Practice: A Clinician's Perspective**	**Dr Tan Lip Kun** Senior Consultant and Medical Director Tissue Engineering & Cell Therapy Laboratory National University Hospital (NUH), Singapore
10.30am	**Panel Session** **"Preparing the healthcare system for Advanced Therapies"**	**Moderator:** **Dr Kellathur Srinivasan** HSA **Panelists:** **Dr Tan Lip Kun** NUH Singapore **Ms Katherine Tsokas** Janssen **Ms Chin Koerner** Novartis
11.15am	**Refreshment Break**	
11.30am	**End of the Module Assessment**	
12.30pm	**Lunch**	
13:00	**Clarifications in EOM Assessment**	CoRE Team
1.30pm	**Regulatory Harmonization and Convergence Initiatives**	**Dr Kellathur Srinivasan** HSA
2.30pm	**Refreshment Break**	
3.15pm	**Upcoming Trends in ATMPs** • Current landscape of ATMPs • Progress and outlook for CART in solid tumours • Innovation to commercialisation of the technologies	**Dr Rahul Pandey** Healthcare Consultant Clarivate Analytics
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**	