GRADUATE CERTIFICATE IN PHARMACEUTICAL REGULATION
GMS5103: Regulation of Cell, Tissue and Gene Therapies

22 June 2020 – 26 June 2020

Venue: Zoom

WORKSHOP PROGRAMME

Learning outcomes

- Define and categorize the scope of ATMPs across different regulatory agencies and list the important features of the regulatory frameworks.
- Describe the critical components in the production and administration of ATMPs including manufacturing, nonclinical and clinical development of the products.
- Become familiar with the premarketing quality and clinical regulatory requirements across ATMP product lifecycle and understand the differences in requirements with respect to conventional pharmaceutical products.
- Recognize the challenges with ATMP benefit-risk assessment and considerations for developing effective post-authorisation measures.
- Identify the challenges in the ATMP product development as well as evaluation and the various initiatives to promote regulatory harmonization.

Graduate Certificate in Pharmaceutical Regulation

GMS5103: Regulation of Cell, Tissue and Gene Therapies

22 – 26 June 2020

Day 1 – 22 June, Mon

	Topic	Speaker/ Organisation
8.00am	**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	**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)
12.30pm	**Lunch**	
1.30pm	**Overview of HSA's CTGTP Regulatory Framework** • Proposed framework for cell, tissue and gene therapy products regulation	**Dr Kellathur Srinivasan** Director Advanced Therapy Products Branch Health Sciences Authority (HSA)
2.30pm	**Refreshment Break**	
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 – 23 June, 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 • Case Example	**Dr Fouad Atouf** Vice President Science—Global Biologics The United States Pharmacopeial Convention (USP)
10.30am	**Utility of Pharmacopoeias** • Engagement with regulators for development of standards	**Dr Fouad Atouf** USP
11.15am	**Refreshment Break**	
11.45am	**Cell and Gene Therapy Manufacturing** • Viral vector production • Ensuring scalability • Cell counting and viability	**Dr Lucas Chan** Co-founder CSO CellVec
12.45pm	**Lunch**	
1.45pm	**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
2.30pm	**ATMP Manufacturing Facility and GMP Inspections** • GMP compliance • Examples of scenarios with non-compliance	**Ms Jessica Teo** Division Director (Audit and Licensing Division) at Health Sciences Authority HPRG
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
5.15pm	**End**	

Day 3 – 24 June, 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	**CMC Considerations and its impact on nonclinical and clinical development of ATMPs** • CMC information requirements • Manufacturing platform and facility considerations • Considerations in nonclinical studies	**Dr Mo Heidaran** Vice President Technical, Regulatory and Technical CMC Paraxel Consulting
Session 4: Clinical Development of ATMPs		
2.00pm	**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
3.00pm	**Refreshment Break**	
3.30pm	**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
4.30pm	**End**	

Day 4 – 25 June, Thurs

	Topic	Speaker/ Organisation
8.30am	**Individual and Group Assessment III**	
9.30am	**Practicum III** • Nonclinical data requirements • Clinical development studies	**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** • Unique characteristics and potential risks of Cell, Tissue and Gene Therapies (CTGTs) • Pharmacovigilance system specific to ATMPs • Post-authorisation safety and efficacy studies	**Ms Christine Ho** Regulatory Consultant Vigilance & Compliance Branch HSA
4.30pm	**Introduction to HSA Cell Therapy Facilities**	**Dr Marieta Chan** Laboratory Director & Consultant Scientist HSA
5.00pm	**End**	

Day 5 – 26 June, Fri

	Topic	Speaker/ Organisation
Session 6: Trends and Opportunities for ATMPs		
8.30am	**Challenges in the Development and Translation of ATMPs**	**Dr Snehal Naik** Regulatory Head Regenerative Medicine & Advanced Therapy Johnson & Johnson
9.30am	**Challenges with Diversified Regulatory Guidelines and Requirements**	**Ms Chin Koerner** Executive Director US Regulatory and Development Policy Novartis
10.30am	**Panel Session** • Q&A with Industry and Regulators	**Moderator:** **Prof John Lim** CoRE **Panelists:** **Dr Snehal Naik** Johnson & Johnson **Ms Chin Koerner** Novartis **Dr Kellathur Srinivasan** HSA
11.00am	**Photo-taking with Faculty and Participants**	**CoRE Team**
11.10am	**Refreshment Break**	
11.30am	**End of the Module Assessment**	**CoRE staff**
12.30pm	**Lunch**	
1.30pm	**Regulatory Harmonization and Convergence Initiatives** • Initiatives undertaken for advanced therapies under auspices of APEC and International Pharmaceutical Regulators Programme	**Dr Kellathur Srinivasan** HSA
2.15pm	**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
2.45pm	**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
4.45 pm	**End**	