Module 22: Regulatory Requirements for Cell Tissue and Gene Therapies

Date: 6 – 8 September 2023

LOCATION: TOPRA OFFICE, LONDON, UK

Module Leader(s): Shaun Stapleton

Day 1: Wednesday 6th September 2023

Time	Activity	Speaker
11.00 11.30	Registration and coffee	

11.30 – 12.30	• Lecture 1: ATMP legislation – an overview
	  • What is an ATMP?: EU/EEA/UK, US, Japan
	  • Legislative framework and key guidance
	  • How does the legislative control vary between regions?
	  • Overlap with blood and tissues legislation
		Alison Wilson, Cell Data Services

12.30 – 13.30	Lunch	

13.30 -14.15	• Lecture 2: ATMP classification and certification procedures
	  • Borderlines between different types of ATMP – the importance of early, correct classification to guide development plans
	  • Procedures to confirm classification in EU and US
	  • Certification procedure in EU
		Daniel Rabbie, Achilles Therapeutics

14:15 – 15:00	• Lecture 3: Drug-device combinations
	  • how ATMP drug device combination products are handled in EU and US
	  • combined ATMPs, interactions with Notified Bodies
	  • regulation of products made from non-viable tissues
	  • interface between tissues and devices
		Shaun Stapleton, ReNeuron

15:00-15:30	Afternoon break	
15:30 – 16:30	• Lecture 4 : Legislation and procedures relating to GMOs	Sabine Ruehle, Boyd Consultants

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Day 2: Thursday 7th September 2023

Time	Activity	Speaker
09:00 – 10:30	• Lecture 5 : Quality/ CMC considerations • Definitions: starting materials, raw materials, DS, DP, and excipients • Control of materials • Cell banking system and testing/specifications • Development of the manufacturing process • Process control (critical quality attributes, critical process parameters and in-process testing). • Overall control of adventitious agents (risk mitigation and testing) • Importance of process and product characterisation • Analytical methods (focus on potency), reference materials and setting specifications. • Stability studies	Christopher Bravery, Advbiols
10.30 – 11:00	Morning break	
11:00 – 12.00	Lecture 5 continued	Christopher Bravery, Advbiols
12.00 – 13.00	Lunch	
13.00 – 15.30	Case study – comparability for ATMPs	Christopher Bravery, Advbiols
15.30 - 16.00	Afternoon Break	
16.00 – 17.00	• Lecture 6: GMP for ATMPs ○ GMP issues specific to ATMPs	Robert Smith, Smiro Qualitas Ltd

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Day 3: Friday 8th September 2023

Time	Activity	Speaker

09.00 – 10.45	• Lecture 7 : Non-Clinical considerations
	  • Overview of key nonclinical studies required by ATMP classification
	  • Key differences relevant to ATMPs compared to biologics and small molecules (e.g. distribution/PK, migration)
	  • EU risk based approach to ATMP development
	  • Challenges with animal and disease models
	  • Toxicology study design and assessment
	  • Non-GLP / GLP requirements
	  • Biodistribution
	  • Tumorigenicity
	  • Immunogenicity
	  • Immunotoxicity
	  • DART
	  • Clinical Translation
	  • There is more than one approach to meet regulatory requirements – comparison of marketed ATMPs.
	  • Supporting information for GMO risk assessments
		Lee Coney,
		Cell and Gene Therapy Catapult

10.45 – 11.15	Morning break	

11.15 – 12:15	• Lecture 8 : Clinical considerations
	  • Challenges of clinical protocol design and consistent clinical procedures, including masking and blinding complications.
	  • Long-term follow-up.
	  • Interface with CMC and nonclinical (e.g. comparability, potency assays)
		Gopalan Narayanan

12:15 - 13.15	Lunch	

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13.15 - 13.45	• Lecture 9: GCP for ATMPs	Celia Gibson, QA Limited

13:45 – 14:30	• Lecture 10 : Expedited programmes and orphan drug issues
	  • US
	  • EU/EEA
	  • Japan
	  • UK
	  • Special considerations for orphan drugs, including challenges of defining same or similar products for ATMPs
		Shaun Stapleton, ReNeuron

14:30-15:00	Afternoon break	
15:00 – 16:30	• Case study – fictional development programme for ATMPs	Shaun Stapleton, ReNeuron
16:30	Close of Module	