Module 22: Regulatory Requirements for Cell Tissue and Gene Therapies

LOCATION: TOPRA OFFICE, LONDON, UK

Module Leader(s): Shaun Stapleton, ReNeuron Limited

Date: 6th - 8th September 2021

Monday 6th September

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 • Agency organisational and review specifics relating to ATMPs – e.g. CAT, OTAT. Commission vs EMA vs MSs responsibilities	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	• **Refreshment Break**	
15:30 – 16:30	• Lecture 4 : Legislation and procedures relating to GMOs	Sabine Ruehle, Boyd Consultants

Module 22: Regulatory Requirements for Cell Tissue and Gene Therapies
Date: **Tuesday 7th September**

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
	  • Comparability considerations during development and post-approval
		Christopher Bravery
		Advbiols

10.30 – 11:00	**Refreshment 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	**Refreshment Break**	
16.00 – 17.00	• Lecture 6 : GMP for ATMPs ○ GMP issues specific to ATMPs	Emma Ewins NSF

Module 22: Regulatory Requirements for Cell Tissue and Gene Therapies
Date: Wednesday 8th September

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	**Refreshment 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**	
13.15 - 13.45	• Lecture 9: GCP for ATMPs	Celia Gibson 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	**Refreshment Break**	
15:00 – 16:30	• Case study – fictional development programme for ATMPs	Shaun Stapleton ReNeuron
16:30	**Close of Module**	