APEC RHSC Centre of Excellence Workshop

Clinical Development and Evaluation of Advanced Therapies

26 & 27 February 2026

Via Zoom

WORKSHOP PROGRAMME

Learning outcomes

At the end of this workshop, participants should be able to

- Explain the environment and challenges in managing Advanced Therapy Medicinal Products (ATMP)
- Describe the life cycle approach to clinical development and the role of non-clinical and clinical investigations.
- Explain the considerations for evaluating benefit-risk profiles with limited databases and appropriate risk management plans for the local population

Target Audience

- Regulatory professionals involved or interested in the regulatory management of cell, tissue and gene therapies, including clinical development, benefit-risk assessment and safety monitoring
- Healthcare professionals, clinical trial professionals, and academic researchers in life sciences who are seeking to better understand the regulatory management of ATMP

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Day 1 – 26 Feb, Thursday

	Topic	Speaker/ Organisation
8.45am	**Welcome**	
Session 1: Clinical Development of ATMP		
9.00am	**Clinical development journey of ATMP** • Overview of ATMP lifecycle • Transition from non-clinical studies to clinical development phase	
9.30am	**Comparison of regulatory frameworks and requirements for clinical development across markets**	
10.15am	**Refreshment Break**	
10.30am	**World Health Organisation's Regulatory framework for ATMP**	
11.00am	**Operational challenges for conducting trials for ATMP**	
Session 2: Clinical Data Evaluation and Statistical Approaches for ATMPs		
11.30am	**Overview of non-clinical evaluation** • Challenges in selecting suitable animal and in vitro models • Different approaches and waivers for non-clinical studies • Biodistribution, persistence and germline integration • Utility of non-clinical conclusions to human studies	
12.30pm	**Lunch**	
1.30pm	**Clinical study designs for ATMP** • Traditional and novel study designs for ATMP • Considerations on small populations for rare diseases • Considerations for endpoints and biomarkers	
2.15pm	**ATMP clinical evaluation** • Challenges for benefit-risk profiling of ATMP • Establishing clinical benefits and managing risks for regulatory decisions	
3.00pm	**Refreshment Break**	
3.30pm	**Statistical interpretation of ATMP clinical data** • Handling endpoint data from novel study designs • Regulatory expectations	
4.15pm	**Case Study** Evaluation of efficacy using non-traditional clinical datasets	
5.30pm	**End of Day 1**	

Day 2 – 27 Feb, Friday

	Topic	Speaker/ Organisation
Session 3: Safety Evaluation and Post-Market Surveillance for ATMPs		
9.00am	**Clinical safety data requirements** • Minimum safety database and SAE criteria • LTFU plans and protocols • References for safety reporting	
9.30am	**Evaluation of safety data for ATMP** • Specific safety concerns associated with ATMP • Data sources and signal detection methodologies • Considerations for recommending post-approval commitments and PBRERs • Design of post-approval studies and use of supportive Real World Evidence (RWE)	
10.15am	**Refreshment Break**	
10.45am	**Challenges in post-market monitoring of ATMP** • Limitations of traditional safety databases • Maintaining patient/disease registries • Causality assessment with delayed onset AEs • Harmonisation across markets	
11.30am	**Case Study** Safety monitoring and RMP for ATMP	
12.30pm	**Lunch**	
Session 4: Regulatory Innovations		
1.30pm	**Using reliance approach for ATMP approvals**	
2.30pm	**RWE in supporting regulatory decision-making – Role in ATMP**	
3.00pm	**Refreshment Break**	
3.30pm	**Exploring new regulatory pathways for rare therapies – UK MHRA**	
4.00pm	**Utility of AI in regulatory processes for novel therapeutics**	
4.30pm	**Panel Discussion** Evolving approaches to facilitate timely access to ATMP	
5.15pm	**Workshop conclusion**	
5.00pm	**End of Workshop**	