Clinical Trials Design for Advanced Therapies (ATMPs) Training Course

Professional Development Training Course Outline

Category	Biotechnology and Pharmaceutical Development	**Duration**	10 Days
Base Fee	$4,000 USD	**Delivery Mode**	Online / On-site Hybrid

Course Introduction

This intensive course offers an essential, deep dive into the specialized discipline of designing and executing clinical trials for Advanced Therapy Medicinal Products. As the convergence of gene therapy, cell therapy, and tissue engineering continues to revolutionize medicine, conventional trial methodologies prove insufficient for these innovative life-saving therapies. ATMPs which include CAR T-cells, in vivo and ex vivo gene vectors, and stem cell products present unique scientific, regulatory, and logistical challenges, such as manufacturing variability, limited patient populations, and the necessity for long-term follow-up

Clinical Trials Design for Advanced Therapies (ATMPs) Training Course directly addresses the critical knowledge gap within the biotech and pharmaceutical sectors. Participants will move beyond traditional frameworks to master adaptive trial designs, Bayesian statistics, and the intricacies of global regulatory bodies, specifically the FDA and EMA. By focusing on patient-centric development and incorporating real-world case studies from the rapidly growing Oncology and Rare Disease pipelines, the course equips professionals with the expertise to navigate the entire translational science lifecycle, ensuring both scientific rigor and Good Clinical Practice (GCP) compliance in this disruptive technology space.

Programme Curriculum

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10 days

Course Objectives

1. Master the Risk-Based Approach to ATMP trial design in alignment with ICH E6(R3) principles.
2. Navigate the complex Global Regulatory Landscape for ATMPs, focusing on FDA IND, EMA PRIME, and Orphan Drug Designation.
3. Optimize trial protocols for Rare Diseases and Small Patient Populations using advanced Bayesian Biostatistics and innovative design
4. Design robust Long-Term Follow-up (LTFU) and Pharmacovigilance strategies unique to gene and cell therapies.
5. Evaluate the critical impact of Chemistry, Manufacturing, and Controls and Product Quality on clinical endpoints and trial variability.
6. Apply current Good Clinical Practice (GCP) for ATMPs to ensure data integrity and patient safety in complex therapeutic areas like CAR T-cell therapy.
7. Select appropriate and clinically meaningful Efficacy Endpoints in early-phase ATMP development.
8. Implement robust Site Selection and Qualification criteria for specialized Advanced Therapy Treatment Centres (ATTCs).
9. Develop effective Patient Recruitment and Retention strategies for geographically dispersed and vulnerable patient cohorts.
10. Assess the Ethical, Legal, and Social Implications (ELSI), including Informed Consent complexities for permanent genetic modification.
11. Interpret and apply Non-Clinical Data to justify First-in-Human (FIH) dosing and escalation in a risk-proportionate manner.
12. Integrate Digital Health Technologies and Decentralized Clinical Trials (DCTs) components into ATMP study operations.
13. Formulate Market Access and Health Technology Assessment (HTA) strategies by collecting essential Health Economic Outcomes Research (HEOR) data.

Target Audience

1. Clinical Development/Trial Managers.
2. Clinical Operations/CRAs
3. Regulatory Affairs Specialists.
4. Biostatisticians/Data Scientists.
5. Quality Assurance (QA) / GMP Professionals.

6. Translational Scientists/Researchers.
7. Pharmacovigilance/Safety Leads.
8. Medical Monitors/Physicians.

Course Modules

Module 1: Foundations of Advanced Therapy Medicinal Products

- Gene Therapy, Somatic Cell Therapy, and Tissue-Engineered Products.
- Understanding the impact of ATMP inherent variability on clinical safety and efficacy.
- Bridging the gap from Non-Clinical Biodistribution and Toxicology data to human First-in-Human protocols.
- Assessing the unique risks of infectivity, immunogenicity, insertional mutagenesis, and vector shedding.
- Allogeneic and Autologous therapies and their logistical/clinical trial implications.
- Case Study: The journey of Zolgensma for Spinal Muscular Atrophy, focusing on non-clinical data translation and dose selection.

Module 2: The Evolving Global Regulatory Framework

- In-depth review of FDA and EMA guidelines and regulatory requirements
- Utilization of FDA RMAT designation, EMA PRIME scheme, and Accelerated Approval for high-unmet need.
- Strategies for pre-IND/Scientific Advice meetings and managing regulatory interaction throughout development.
- Integrating GMP requirements for ATMP manufacturing into the clinical trial application
- Structuring the Investigational New Drug or Clinical Trial Application with a focus on ATMP-specific sections.
- Case Study: Comparison of the US and EU regulatory pathway for a licensed CAR T-cell therapy

Module 3: Innovative Trial Design for Small Patient Populations

- Principles of Bayesian statistics for dose-finding and early efficacy signals.
- Implementation of Seamless Phase I/II and Adaptive Randomization to accelerate development.
- Designing Basket, Umbrella, and Platform Trials to efficiently study multiple indications or products simultaneously.
- Addressing ethical and practical challenges in selecting appropriate control arms, especially for Rare/Orphan Diseases.
- Structuring exploratory and confirmatory phases to satisfy both regulatory and statistical requirements.
- Case Study: The design of a platform trial for a novel CRISPR-based in vivo gene-editing product for multiple congenital disorders.

Module 4: Endpoints, Outcomes, and Data Analysis

- Selection of appropriate and persuasive efficacy endpoints
- Regulatory acceptance criteria and validation of surrogate markers in early ATMP trials.
- Health Economic Outcomes Research.
- Establishing appropriate Data Monitoring Committees and Data and Safety Monitoring Boards roles.
- Planning for interim analyses and potential early stopping rules in high-risk/high-reward trials.
- Case Study: Analyzing the selection and regulatory acceptance of vector copy number as a proxy for efficacy in a specific in vivo gene therapy.

Module 5: Patient Safety and Pharmacovigilance

- Detailed understanding and grading of unique adverse events
- Establishing rigorous SAE/SUSAR reporting processes specific to ATMP-induced toxicities.
- Long-Term Follow-up Protocols.
- Utilizing registries and observational studies to complement trial data and track real-world safety profiles.
- Risk Evaluation and Mitigation Strategies.
- Case Study: Protocol development for tracking and managing potential long-term malignancy risk following a specific lentiviral vector gene therapy.

Module 6: CMC and Product Logistics in Clinical Trials

- Overview of GMP/GTP requirements and the criticality of phase-appropriate manufacturing scale-up.
- Challenges and regulatory expectations for Lot Release Criteria and product quality testing during a clinical trial.
- Implementing robust tracking and security for personalized therapies.
- Designing the cold chain logistics for cryopreserved or fresh products from vein-to-vein/biopsy-to-patient.
- Ensuring the Investigational Medicinal Product Dossier accurately reflects product changes and manufacturing control.
- Case Study: Mapping the logistical and documentation flow for an autologous T-cell therapy trial, from leukapheresis to infusion.

Module 7: Clinical Operations and Site Management

- Criteria for selecting and qualifying specialized Advanced Therapy Treatment Centres with required infrastructure.
- Ensuring site personnel are trained in product handling, administration, and acute toxicity management.
- Strategies for managing the high complexity and low tolerance for deviation in ATMP trial protocols.
- Implementing a focused Risk-Based Quality Management strategy to prioritize critical data points and processes.
- Preparing for regulatory inspections with a focus on GCP compliance for ATMP-specific documentation.
- Case Study: Developing a site initiation plan checklist and required capabilities matrix for a Phase III Tissue-Engineered Product trial.

Module 8: Patient Recruitment, Ethics, and Informed Consent

- Incorporating the patient and caregiver voice into protocol development to enhance feasibility and adherence.
- Overcoming barriers in recruiting patients for ultra-rare diseases or in competitive therapeutic areas
- Managing the complexity of the consent process for high-risk, potentially permanent gene/cell modification.
- Specific ethical and regulatory considerations for pediatric patients, especially in genetic disorders.
- Strategies to ensure diverse patient representation in ATMP trials, in line with emerging global mandates.
- Case Study: Review of an ethical dilemma concerning the re-consent process for Long-Term Follow-up after an initial gene therapy trial.

Module 9: Biologics and Device Combination Products

- Understanding when an ATMP becomes a Combination Product
- Navigating the joint regulation by FDA CBER/CDRH or equivalent joint-review processes in the EU.

- Integrating ISO 14155/GCP for the device component into the overall ATMP clinical protocol.
- Performing a holistic risk assessment for the combined product, focusing on device-product interaction.
- Ensuring comprehensive Instructions for Use and appropriate labeling for the combined product during the trial.
- Case Study: Regulatory considerations and trial design for a tissue-engineered scaffold combined with patient-derived cells.

Module 10: Gene Therapy Trial Design

- Comparing trial designs for different vector types and their associated biodistribution risks.
- Specific strategies for dose-finding in in vivo gene therapy, considering translational data limitations and vector exposure.
- Designing assays and monitoring strategies to detect and manage host immune response to the vector/product.
- Protocol requirements for testing and counseling related to potential germline transmission.
- Clinical trial planning for CRISPR/TALEN-based therapies, focusing on off-target effects and in vivo delivery.
- Case Study: Designing a FIH study for a novel AAV vector gene therapy targeting a rare liver disorder, focusing on liver toxicity monitoring.

Module 11: Cell Therapy Trial Design

- Managing the clinical implications of variability in cell source, processing, and viability on patient outcome.
- Rationale for dosing based on cell number, viability, and potency assays for adoptive transfer therapies.
- Establishing clinical pathways for acute toxicities like Cytokine Release Syndrome and ICANS in CAR T-trials.
- Protocol strategies for patient management following an Out-of-Specification or manufacturing failure.
- Integrating research biopsies and biological sample collection into the trial schedule to assess persistence and mechanism of action.
- Case Study: The clinical design and safety monitoring structure for a multi-site Phase II CAR T-cell trial in refractory hematological malignancy.

Module 12: Data Management and Digital Innovation

- Implementing CDISC standards and ATMP-specific data models for clinical and manufacturing data.
- Managing the complex, often disparate datasets from the manufacturing side, the clinical side, and long-term follow-up registries.
- Feasibility and implementation of remote monitoring, e-Consent, and at-home sample collection for ATMPs.
- Utilization of wearables and mobile apps for continuous, remote monitoring of patient safety and quality of life.
- Ensuring complete and validated electronic audit trails for all critical ATMP processes
- Case Study: Implementing a Decentralized Clinical Trial component for a European ATMP trial involving remote safety checks and e-LTFU consent.

Module 13: Financial, Access, and Commercialization Strategy

- Understanding the high cost of goods and the economic implications for trial budgeting and eventual pricing strategy.

- Integrating trial design with the evidence requirements of payers and Health Technology Assessment bodies.
- Utilizing mechanisms like Conditional Marketing Authorization or Expanded Access Programs
- Basic considerations for protecting the novel ATMP and related processes during clinical development.
- Planning for the transition from clinical-scale to commercial-scale production and its impact on the MAA.
- Case Study: Developing an HEOR plan to support the value proposition for a breakthrough stem cell therapy requiring a high upfront cost.

Module 14: Quality Systems and Auditing for ATMPs

- Defining roles and responsibilities at the critical junction between clinical research and manufacturing quality.
- Tailoring the Quality Management System for the phase of the trial
- Rigorous qualification and management of specialized vendors
- Standardizing investigation and resolution of deviations related to product quality and clinical administration.
- Conducting internal mock inspections focused on high-risk ATMP areas
- Case Study: Root cause analysis and corrective action plan for a critical protocol deviation involving ATMP product temperature excursion.

Module 15: Emerging Trends and Future Directions

- Overview of Gene Editing, Induced Pluripotent Stem Cells, and mRNA therapies in the clinical pipeline.
- Application of Artificial Intelligence and Machine Learning in patient selection and predictive safety modeling.
- Discussion on the role of ATMP Networks and harmonizing standards globally.
- Debates and regulatory perspectives on ATMPs moving toward human enhancement or prophylactic use.
- Future outlook on regulatory convergence
- Case Study: The ethical and practical design challenges for a first-in-human clinical trial of a novel in vivo Base Editing therapy.

Training Methodology

The course employs a blended, highly interactive methodology to ensure practical mastery of complex topics:

1. Expert-Led Lectures
2. Real-World Case Studies.
3. Interactive Workshops/Group Exercises
4. Regulatory Simulation.
5. Q&A/Expert Panels.

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Certification

Upon successful completion of this training, participants will be issued with a globally- recognized certificate.

Tailor-Made Course

We also offer tailor-made courses based on your needs.

Key Notes

a. The participant must be conversant with English.

b. Upon completion of training the participant will be issued with an Authorized Training Certificate

c. Course duration is flexible and the contents can be modified to fit any number of days.

d. The course fee includes facilitation training materials, 2 coffee breaks, buffet lunch and A Certificate upon successful completion of Training.

e. One-year post-training support Consultation and Coaching provided after the course.

f. Payment should be done at least a week before commence of the training, to FINESKILL TRAINING CENTER account, as indicated in the invoice so as to enable us prepare better for you.

Upcoming Scheduled Sessions

Start Date	End Date	Location	Price
07 Sep 2026	18 Sep 2026	Online	$4,000
14 Sep 2026	25 Sep 2026	Online	$4,000
21 Sep 2026	02 Oct 2026	Online	$4,000
28 Sep 2026	09 Oct 2026	Online	$4,000
05 Oct 2026	16 Oct 2026	Online	$4,000
12 Oct 2026	23 Oct 2026	Online	$4,000
19 Oct 2026	30 Oct 2026	Online	$4,000
26 Oct 2026	06 Nov 2026	Online	$4,000

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