Ex Vivo vs In Vivo Gene Therapy Strategies 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

The field of gene therapy is revolutionizing modern medicine, offering curative potential for previously untreatable genetic disorders. As genomic technologies rapidly evolve, professionals in biotechnology, pharmacology, and medical research must stay updated on the latest strategies in Ex Vivo and In Vivo gene therapy. Ex Vivo vs In Vivo Gene Therapy Strategies Training Course provides a deep-dive into delivery systems, vector optimization, gene editing tools, regulatory frameworks, and clinical trial design for both therapeutic approaches. Participants will explore the molecular basis, process pipelines, and comparative efficacy of Ex Vivo and In Vivo gene therapy strategies through real-world case studies and industry applications.

Designed with a future-focused perspective, this course is ideal for professionals looking to enhance their knowledge in CRISPR-Cas9 systems, AAV vectors, CAR-T cell therapy, viral and non-viral delivery, and genomic medicine innovation. With interactive modules and expert-led sessions, learners will master both foundational concepts and advanced applications. Whether your part of a clinical development team or leading innovation in biotech R&D, this course bridges the gap between theory and translational impact.

Programme Curriculum

Ex Vivo vs In Vivo Gene Therapy Strategies Training Course

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Course Duration

10 days

Course Objectives

1. Understand the principles of gene therapy and delivery mechanisms.
2. Differentiate between Ex Vivo and In Vivo strategies.
3. Master CRISPR-Cas9 and emerging gene editing technologies.
4. Analyze viral vs non-viral vector systems.
5. Examine AAV, lentiviral, and retroviral vectors in therapy.
6. Evaluate preclinical models and animal studies for gene therapy.
7. Understand clinical trial phases for gene therapy products.
8. Assess manufacturing challenges and scale-up processes.
9. Interpret regulatory guidelines from the FDA and EMA.
10. Explore immune response challenges in gene therapy.
11. Identify targeted delivery techniques for genetic payloads.
12. Learn from case studies in rare diseases and oncology.
13. Gain insights into future trends in genomic medicine.

Target Audience

1. Biomedical Scientists
2. Clinical Research Associates
3. Biotech & Pharma Professionals
4. Medical Doctors & Oncologists
5. Regulatory Affairs Specialists
6. Molecular Biologists
7. Gene Therapy Startups & Entrepreneurs
8. Academic Researchers & Postdocs

Course Modules

Module 1: Introduction to Gene Therapy

- Historical evolution of gene therapy
- Types of gene therapy (somatic vs germline)
- DNA, RNA-based therapeutics
- Overview of gene transfer technologies

- **Case Study:** SCID (Severe Combined Immunodeficiency)

Module 2: Fundamentals of Ex Vivo Gene Therapy

- Definition and workflow
- Cell isolation and genetic modification
- CAR-T therapy as a model
- Safety and efficacy considerations
- **Case Study:** CAR-T in hematologic cancers

Module 3: Fundamentals of In Vivo Gene Therapy

- Direct gene delivery mechanisms
- Tissue targeting and specificity
- Common vectors used
- Challenges and immune responses
- **Case Study:** AAV in spinal muscular atrophy

Module 4: Viral Vector Technologies

- Lentivirus, retrovirus, and AAV comparison
- Packaging and production
- Tropism and transduction efficiency
- Vector safety modifications
- **Case Study:** Hemophilia B treatment with AAV

Module 5: Non-Viral Vector Systems

- Lipid nanoparticles (LNPs)
- Electroporation and plasmid delivery
- Nanoparticles in CRISPR delivery
- Advantages and limitations
- **Case Study:** COVID-19 mRNA vaccine platforms

Module 6: CRISPR-Cas Gene Editing

- CRISPR-Cas9, Cas12, and Cas13 overview
- Editing precision and off-target effects
- Delivery in Ex Vivo and In Vivo contexts
- Emerging tools like base and prime editing
- **Case Study:** Sickle Cell Disease therapy

Module 7: CAR-T and Cell Therapy Platforms

- CAR-T design and engineering
- T cell expansion and modification
- Quality control in Ex Vivo processing
- Clinical outcomes and FDA approvals
- **Case Study:** Kymriah vs Yescarta comparison

Module 8: Regulatory Framework

- FDA and EMA approval pathways

- IND and BLA applications
- Gene therapy product classification
- Risk assessment protocols
- **Case Study:** Zolgensma approval process

Module 9: Manufacturing and Scale-Up

- GMP in gene therapy production
- Cell banking and expansion
- Vector manufacturing bottlenecks
- Cost and scalability considerations
- **Case Study:** Bluebird Bio vector production

Module 10: Preclinical and Clinical Trials

- Designing preclinical studies
- Translating animal models to humans
- Phase I-III trials for gene therapy
- Safety and efficacy endpoints
- **Case Study:** Luxturna trial design

Module 11: Immune Response Management

- Host immune system interactions
- Strategies to mitigate immune reactions
- Use of immunosuppressive agents
- Re-dosing limitations
- **Case Study:** AAV immunogenicity in DMD

Module 12: Targeted Delivery Strategies

- Organ-specific delivery
- Blood-brain barrier penetration
- Muscle and ocular targeting
- Use of tissue-specific promoters
- **Case Study:** CNS delivery in Batten disease

Module 13: Ethical and Safety Concerns

- Germline editing controversies
- Informed consent in gene trials
- Long-term follow-up requirements
- Ethical use of CRISPR
- **Case Study:** He Jiankui incident

Module 14: Trends and Innovation in Gene Therapy

- Artificial intelligence in gene therapy design
- Next-gen delivery platforms
- Synthetic biology in vector design
- Global market trends and investment
- **Case Study:** Biotech pipeline analysis (2025)

Module 15: Capstone Integration & Assessment

- Comparative analysis: Ex Vivo vs In Vivo
- Personalized treatment planning
- Project simulation: gene therapy design
- Real-world problem-solving
- **Case Study:** Student-led mock trial and therapy design

Training Methodology

This course employs a participatory and hands-on approach to ensure practical learning, including:

- Interactive lectures and presentations.
- Group discussions and brainstorming sessions.
- Hands-on exercises using real-world datasets.
- Role-playing and scenario-based simulations.
- Analysis of case studies to bridge theory and practice.
- Peer-to-peer learning and networking.
- Expert-led Q&A sessions.
- Continuous feedback and personalized guidance.

Register as a group from 3 participants for a Discount

Send us an email: info@fineskilltrainingcenter.org or call +254769199797

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
23 Nov 2026	04 Dec 2026	Online	$2,000
23 Nov 2026	04 Dec 2026	Physical	$3,000
23 Nov 2026	04 Dec 2026	Physical	$6,000
23 Nov 2026	04 Dec 2026	Physical	$6,000
23 Nov 2026	04 Dec 2026	Physical	$12,000
23 Nov 2026	04 Dec 2026	Physical	$9,000
23 Nov 2026	04 Dec 2026	Physical	$12,000
23 Nov 2026	04 Dec 2026	Physical	$12,000

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