CRISPR-Cas9 Technology - Advanced Applications and Ethics 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 CRISPR-Cas9 system has permanently revolutionized genome editing, offering unprecedented precision medicine capabilities across biotechnology, agriculture, and therapeutics. This powerful, flexible technology enables accurate gene modification, accelerating the development of treatments for complex genetic disorders like sickle cell disease, beta-thalassemia, and cancer. Beyond foundational research and disease modeling, the field is rapidly advancing to next-generation techniques such as Prime Editing and Base Editing, which minimize off-target effects and promise to correct a vast number of human genetic variants. Mastering these advanced concepts and practical applications is now essential for any professional navigating the frontier of synthetic biology and functional genomics.

CRISPR-Cas9 Technology - Advanced Applications and Ethics Training Course provides a critical, dual focus: equipping participants with technical mastery in cutting-edge CRISPR variants and delivery systems, while simultaneously cultivating a deep understanding of the complex bioethics and regulatory frameworks necessary for responsible innovation. We bridge the gap between bench-side protocols such as designing sgRNAs and executing gene knockouts and the crucial societal debates surrounding human germline editing, genetic enhancement, and ensuring equitable access to CRISPR therapies. Graduates will be poised to lead research, drive commercial development, and champion the global conversation on the future of DNA-based diagnostics and regenerative medicine.

Programme Curriculum

CRISPR-Cas9 Technology - Advanced Applications and Ethics Training Course

Introduction

The CRISPR-Cas9 system has permanently revolutionized genome editing, offering unprecedented precision medicine capabilities across biotechnology, agriculture, and therapeutics. This powerful, flexible technology enables accurate gene modification, accelerating the development of treatments for complex genetic disorders like sickle cell disease, beta-thalassemia, and cancer. Beyond foundational research and disease modeling, the field is rapidly advancing to next-generation techniques such as Prime Editing and Base Editing, which minimize off-target effects and promise to correct a vast number of human genetic variants. Mastering these advanced concepts and practical applications is now essential for any professional navigating the frontier of synthetic biology and functional genomics.

CRISPR-Cas9 Technology - Advanced Applications and Ethics Training Course provides a critical, dual focus: equipping participants with technical mastery in cutting-edge CRISPR variants and delivery systems, while simultaneously cultivating a deep understanding of the complex bioethics and regulatory frameworks necessary for responsible innovation. We bridge the gap between bench-side protocols such as designing sgRNAs and executing gene knockouts and the crucial societal debates surrounding human germline editing, genetic enhancement, and ensuring equitable access to CRISPR therapies. Graduates will be poised to lead research, drive commercial development, and champion the global conversation on the future of DNA-based diagnostics and regenerative medicine.

Course Duration

10 days

Course Objectives

Upon completion, participants will be able to:

1. Master the mechanism and application of Prime Editing and Base Editing for precise single-base corrections.
2. Design and optimize highly specific sgRNAs and Cas enzyme variants to minimize off-target effects.
3. Evaluate and select appropriate delivery mechanisms, including AAV vectors, lipid nanoparticles, and RNP complexes for in vivo and ex vivo applications.
4. Analyze the latest CRISPR-based diagnostics for rapid and multiplexed viral/bacterial detection.
5. Develop robust disease models using multiplex gene editing strategies.
6. Apply CRISPR tools for functional genomics and high-throughput cancer dependency screens.
7. Critically assess the potential of CRISPR for cancer immunotherapy, specifically in generating next-generation CAR T-cells.
8. Formulate strategies for using CRISPR Gene Drives and evaluate their ecological risks in environmental and public health contexts
9. Analyze the clinical trial landscape for CRISPR therapies
10. Discuss the profound ethical dilemmas of human germline editing and the concept of "designer babies."
11. Interpret current global regulatory frameworks for the clinical translation of genome-edited products.
12. Address issues of genetic equity and access to therapy in low-resource settings.
13. Troubleshoot common experimental failures, including low editing efficiency and analysis of mosaicism using next-generation sequencing (NGS) data.

Target Audience

1. Biomedical Researchers.
2. Biotechnology & Pharmaceutical Scientists.
3. Molecular Biologists and Geneticists.
4. Principal Investigators (PIs) and Lab Managers.

5. Bioinformatics Specialists.
6. Regulatory Affairs Professionals and Bioethicists.
7. Clinical Researchers
8. Advanced Graduate Students in Genetics, Molecular Biology, and Synthetic Biology.

Course Modules

Module 1: Foundational Mechanisms and CRISPR System Diversity

- Review of the prokaryotic defense system.
- Mechanisms of DNA repair
- Exploring Cas enzyme diversity.
- High-fidelity variants
- Case Study: The Nobel Prize-winning discovery of Cas9.

Module 2: Advanced Gene Editing Tools (Base & Prime)

- Mechanism of Base Editing
- Mechanism of Prime Editing
- Design principles and limitations for Base and Prime Editors compared to traditional Cas9.
- Analyzing the scope of correction.
- Case Study: Application of Base Editing to correct the HBB-E6V mutation for Sickle Cell Disease

Module 3: Vector Delivery Systems & In Vivo Gene Editing

- Fundamentals of CRISPR delivery
- Optimizing Adeno-Associated Virus serotypes for tissue-specific delivery
- Delivery of Ribonucleoprotein complexes via electroporation for transient, low-toxicity editing.
- Strategies for overcoming immunogenicity and maximizing cargo capacity in viral vectors.
- Case Study: Clinical translation of AAV-CRISPR for in vivo editing of the TTR gene to treat transthyretin amyloidosis.

Module 4: Functional Genomics and High-Throughput Screening

- Introduction to CRISPR-KO and CRISPR-a/CRISPR-i systems for gene modulation.
- Design and execution of whole-genome CRISPR loss-of-function screens in cell culture models.
- Bioinformatic analysis of screening data.
- Introduction to Multiplex Gene Editing for simultaneously targeting multiple genes and pathways.
- Case Study: Using a CRISPR-a screen to identify novel resistance mechanisms to chemotherapy drugs in lung cancer cell lines.

Module 5: CRISPR in Cancer Immunotherapy

- Engineering CAR T-cells with CRISPR.
- Targeting immune checkpoint inhibitors using gene knockout to boost anti-tumor activity.
- Strategies for generating allogeneic T-cell therapies to improve accessibility and scalability.
- Delivery and validation of CRISPR-edited T-cells for clinical application.
- Case Study: Phase 1 trial data on CRISPR-edited PD-1 knockout T-cells for treating refractory metastatic non-small cell lung cancer.

Module 6: Therapeutic Applications in Hematopoietic Diseases

- Targeting the Bcl11a erythroid enhancer to reactivate fetal hemoglobin production.
- Ex vivo editing of Hematopoietic Stem Cells for treating Sickle Cell Disease and Beta-thalassemia.
- Methodology for HSPC mobilization, harvest, editing via RNP, and autologous transplantation.
- Long-term engraftment and monitoring of corrected cell populations.
- Case Study: Analyzing the groundbreaking success and long-term safety data from the first FDA-approved CRISPR therapy for Sickle Cell Disease

Module 7: CRISPR-Based Diagnostics

- The mechanism of CRISPR-Cas13 for highly sensitive and specific detection.
- Protocols for the SHERLOCK and DETECTR platforms.
- Development of rapid, low-cost, and portable Point-of-Care diagnostics.
- Designing assays to simultaneously detect multiple pathogens or resistance genes.
- Case Study: The adaptation of CRISPR-Cas13 for rapid, sensitive, and low-cost diagnosis of SARS-CoV-2 and its variants during a pandemic.

Module 8: Design and Validation Protocols

- Computational sgRNA design tools and rules for maximizing on-target efficiency.
- Predicting and ranking off-target sites and experimental techniques for validation.
- Wet-lab validation protocols.
- Advanced quantitative analysis using Next-Generation Sequencing data and bioinformatic pipelines
- Case Study: A protocol for designing and validating a highly specific gRNA to knock out the CCR5 receptor in human T-cells for HIV resistance research.

Module 9: Agricultural and Food Security Applications

- Application of CRISPR to create disease-resistant crops
- Engineering livestock for enhanced traits
- Accelerating traditional breeding cycles using precise, non-transgenic CRISPR modifications.
- Understanding the different global policies for "gene-edited" and "GMO" products.
- Case Study: Using CRISPR to silence a gene in Cassava to reduce its natural cyanide content, enhancing food safety in sub-Saharan Africa.

Module 10: Environmental and Gene Drive Applications

- Mechanism and potential of CRISPR Gene Drives for species-wide genetic modification.
- Applications in public health: Controlling mosquito-borne diseases by driving sterility or trait resistance.
- Technical safeguards and ethical considerations for preventing "technology escape" and irreversible changes.
- Assessing the long-term impact of a gene drive on biodiversity and non-target species.
- Case Study: Modeling the potential release of CRISPR-edited Anopheles mosquitoes into a wild population to prevent the transmission of the Plasmodium falciparum parasite.

Module 11: Bioethics and Societal Implications

- Defining the ethical boundary between somatic and germline editing.
- Frameworks for Informed Consent in patients undergoing experimental CRISPR treatments.
- The ethical debate over risk, benefit for life-threatening and less severe genetic conditions.
- Addressing issues of genetic inequality and ensuring equitable access to costly CRISPR-based therapies.

- Case Study: Ethical review of the first in vivo CRISPR clinical trial for Leber Congenital Amaurosis, a form of blindness, and the acceptable level of therapeutic risk.

Module 12: Bioethics and Societal Implications

- The profound controversy of Human Germline Editing and the creation of "designer babies."
- Arguments for and against heritable gene editing
- Philosophical concepts: Distinguishing between disease treatment/prevention and genetic enhancement.
- The socio-cultural impact of eliminating or editing disability-associated genes.
- Case Study: Detailed analysis of the He Jiankui case, the global scientific response, and the immediate impact on international regulatory bodies.

Module 13: Global Regulatory and Policy Landscape

- Overview of major global regulatory bodies.
- The regulatory pathway for Investigational New Drug and Biologics License Application for gene therapies.
- Current global moratoriums and varying national laws on germline editing.
- The complexity of Intellectual Property and patent disputes in the CRISPR field.
- Case Study: Comparing the regulatory approval process in the US versus the UK for the first approved ex vivo CRISPR therapy

Module 14: CRISPR in Model Organisms and Cell Engineering

- Techniques for generating CRISPR-edited animal models for pre-clinical research.
- Developing Induced Pluripotent Stem Cells and organoids as patient-specific disease models using CRISPR.
- Advanced cell line engineering.
- The use of dCas9 for targeted DNA labeling and imaging.
- Case Study: Using CRISPR to engineer human iPSCs from Parkinson's disease patients to introduce or correct mutations for drug screening.

Module 15: The Future of Genome Engineering

- Next-generation Cas enzymes and systems
- Emerging applications in Synthetic Biology.
- The intersection of AI and CRISPR.
- Ethical and technological horizon scanning.
- Case Study: The potential future use of CRISPR to target and eliminate antibiotic-resistant genes in the human gut microbiome.

Training Methodology

The course employs a blended and highly practical methodology designed for deep learning and skill translation:

1. Interactive Lecture & Seminar Discussions.
2. Hands-on Bioinformatics Workshops
3. Virtual Lab Simulations/Protocols.
4. Case Study & Policy Debates.
5. Final Project.

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

Ready to enroll or request a customized program? Book online or contact us:

Register Online Now

Email: info@fineskilltrainingcenter.com | Website: www.fineskilltrainingcenter.com