Advanced Gene Editing Beyond CRISPR (Base/Prime Editing) 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 genetic engineering is undergoing a profound revolution, rapidly moving past the limitations of first-generation CRISPR-Cas9 to embrace ultra-precise and safer new tools. This advanced training course is dedicated to mastering the next-generation of genome editing technology, namely Base Editing (BE) and Prime Editing (PE). These CRISPR-derived systems enable single-nucleotide correction and small insertion/deletion (indel) modifications without creating the error-prone double-strand DNA breaks (DSBs) that plague traditional methods. Participants will gain a deep, functional understanding of the molecular mechanisms, strategic design principles, and complex bioinformatics workflows necessary to implement these technologies across therapeutic development, synthetic biology, and precision agriculture. This is an indispensable course for scientists and industry professionals aiming to lead the charge in in vivo gene therapy and personalized medicine.

Advanced Gene Editing Beyond CRISPR (Base/Prime Editing) Training Course offers both the theoretical foundation and hands-on practical expertise required for cutting-edge research and biotechnology application. You will learn to design, optimize, and execute Base and Prime Editing experiments, focusing on crucial aspects like guide RNA selection, delivery system optimization, and rigorous off-target effect analysis. Through immersive case study analysis and project-based learning, the course directly addresses real-world challenges, including complex gene correction strategies and the translational science pipeline. By completing this training, you will be proficient in utilizing these programmable nucleases to accelerate drug discovery, engineer advanced cell models, and navigate the evolving regulatory landscape of these powerful precision editing tools.

Programme Curriculum

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Course Objectives

Upon completion of this course, participants will be able to:

1. Differentiate the molecular mechanisms of Base Editing and Prime Editing (PE).
2. Master the rational design and optimization of pegRNAs and sgRNAs for BE and PE.
3. Analyze the advantages and limitations of BE and PE compared to conventional CRISPR-Cas9 and HDR.
4. Execute laboratory protocols for efficient delivery of advanced editors using AAV vectors and Lipid Nanoparticles (LNPs).
5. Critically Evaluate and minimize off-target effects and bystander editing in both BE and PE systems.
6. Utilize advanced bioinformatics tools for target site selection, guide RNA scoring, and edit prediction.
7. Design complex gene correction strategies for monogenic disorders and challenging genomic loci.
8. Quantify editing efficiency and specificity using gold-standard methods like ddPCR and Next-Generation Sequencing (NGS).
9. Develop robust cell line engineering and disease modeling platforms using precision editing.
10. Explore the therapeutic potential and translational science pipeline for in vivo and ex vivo therapies.
11. Assess the latest developments in next-generation editors, including CRISPR-free systems and AI-driven design.
12. Address the bioethical implications and navigating the regulatory landscape of germline and somatic editing.
13. Troubleshoot experimental challenges related to editor expression, toxicity, and cell-type specificity.

Target Audience

1. Research Scientists/Associates in Biotech/Pharma R&D
2. Postdoctoral Researchers and PhD Candidates in Genomics and Molecular Biology
3. Principal Investigators (PIs) seeking to integrate advanced editing into their labs
4. Clinical/Translational Scientists focused on gene therapy development
5. Core Facility Staff managing genome editing services
6. Bioinformatics Specialists involved in guide RNA design and NGS data analysis
7. Venture Capitalists and Biotech Investors requiring deep technical due diligence
8. Regulatory Affairs Professionals dealing with gene editing product approvals

Course Modules

Module 1: CRISPR-Cas9 Foundations and Limitations

- Mechanism, components, and primary applications.
- Challenges of DSBs and Homology-Directed Repair (HDR).
- The inevitability of indels and their clinical implications.
- Case Study: CRISPR/Cas9 Phase 1 trial for Sickle Cell Disease and the reliance on HDR efficiency.
- The imperative for precision editing beyond Cas9.

Module 2: Introduction to Base Editing (BE)

- Architecture of Base Editors.
- Cytosine Base Editors (CBE) vs. Adenine Base Editors (ABE).
- Understanding the editing window and bystander effects.
- Case Study: The use of ABE to correct the G-to-A mutation causing Hereditary Hemochromatosis.
- Design principles for BE guide RNAs.

Module 3: Introduction to Prime Editing (PE)

- Architecture of Prime Editors
- Mechanism of prime editing guide RNA and nick-site selection.
- PE for precise substitutions, insertions, and small deletions.
- Case Study: PE application for a F5 gene insertion to treat a form of hemophilia.
- Comparison of PE vs. HDR for knock-in strategies.

Module 4: Advanced Editor Design and Optimization

- Engineering enhanced editors.
- Optimizing editor expression and reducing cellular toxicity.
- Strategic selection between Base Editing vs. Prime Editing for a target.
- Case Study: Directed evolution of a new ABE for improved specificity and activity.
- Enhancing PE efficiency with Prime Editor 3 (PE3) and 4 (PE4) strategies.

Module 5: Delivery Systems for In Vitro and In Vivo Use

- Delivery of DNA vs. RNA-protein (RNP) complexes.
- Viral vectors: Adeno-Associated Virus (AAV) packaging and tropism selection.
- Non-viral methods: Lipid Nanoparticles (LNPs) and electroporation.
- Case Study: LNP delivery of mRNA-encoded Base Editors for in vivo liver editing.
- Considerations for tissue-specific delivery and immunogenicity.

Module 6: Bioinformatics for Target Selection

- Algorithms for identifying optimal BE and PE target sites.
- Tools for gRNA and pegRNA design, scoring, and specificity prediction.
- Predicting and quantifying potential off-target sites using computational methods.
- Case Study: Utilizing Deep-Learning models to predict Base Editor activity and specificity.
- Automation of high-throughput target library generation.

Module 7: Experimental Execution and Cell Line Models

- Detailed protocols for mammalian cell culture and transfection.
- Cloning and assembly of Base and Prime Editing components.
- Strategies for generating and validating stable, edited cell lines.
- Case Study: Creating an isogenic human iPSC model for a neurological disorder using Prime Editing.
- Troubleshooting low editing efficiency and cell death.

Module 8: Off-Target Analysis and Quality Control

- Methods for detecting off-target edits
- Next-Generation Sequencing (NGS) for deep sequencing of target loci.
- Quantifying on-target vs. off-target ratios and assessing edit purity.
- Case Study: Evaluating a high-fidelity Base Editor variant with minimal in vivo off-targets.
- Establishing quality assurance protocols for edited cells.

Module 9: Quantitative Analysis with NGS and ddPCR

- Droplet Digital PCR (ddPCR) for precise editing frequency measurement.
- NGS data processing.
- Interpreting complex Prime Editing indel/substitution data.
- Case Study: Statistical analysis of Base Editing results from a pooled library screen.
- Bioinformatics pipelines for automated edit quantification.

Module 10: Applications in Disease Modeling

- Using BE/PE to create point mutations that mimic human disease.
- Engineering reporter cell lines and organoids for high-throughput screening.
- Generating knockout/knock-in models with unprecedented accuracy.
- Case Study: Correcting the CFTR mutation in patient-derived intestinal organoids using Base Editing.
- Models for polygenic disorders and complex trait studies.

Module 11: Therapeutic Development and Ex Vivo Therapy

- Ex vivo editing of Hematopoietic Stem Cells (HSCs) and T-cells
- Optimizing editing for clinical-grade cell product manufacturing.
- Scale-up and process development for cell therapy trials.
- Case Study: Developing a Prime Editing strategy for a gene correction in CAR-T cells to prevent exhaustion.
- Regulatory requirements for ex vivo gene-edited cell products.

Module 12: In Vivo Gene Therapy Applications

- Strategies for liver, eye, and muscle in vivo editing.

- Dose-response, toxicity, and persistence of delivered editors.
- Overcoming immune response to viral and protein components.
- Case Study: Pre-clinical data on in vivo Base Editing in the liver to treat Familial Hypercholesterolemia (FH).
- Challenges of editing non-dividing cells

Module 13: Advanced and Emerging Systems

- CRISPR-free systems.
- RNA editing platforms.
- Directed evolution and AI-driven protein design for new editors.
- Case Study: A new Base Editor designed using Generative AI with superior A>G conversion efficiency.
- The future of multiplexed editing for complex traits.

Module 14: Regulatory and Ethical Landscape

- FDA/EMA guidelines for gene-edited products and clinical trials.
- The bioethical debate surrounding germline vs. somatic cell editing.
- Informed consent and patient considerations in clinical trials.
- Case Study: The global regulatory response to the first human germline editing events.
- Intellectual Property (IP) issues and licensing in the advanced editing space.

Module 15: Course Capstone and Project Design

- Integrated design of a complete BE or PE project from target identification to validation.
- Developing a translational strategy and funding proposal outline.
- Peer review and expert feedback on project designs.
- Case Study: Final project presentation: Designing a Base Editing therapeutic for Duchenne Muscular Dystrophy (DMD) exon skipping.
- Q&A and career paths in advanced genomics.

Training Methodology

The course employs a high-intensity, blended learning approach to ensure deep conceptual mastery and practical skill acquisition:

- Interactive Lectures & Seminars.
- Bioinformatics Workshops.
- Wet-Lab Demonstrations/Protocols.
- Case Study-Based Learning.
- Project-Based Module.

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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