
HMX Pro Genetics - Nucleic Acid Therapeutics

Even with the thousands of approved small molecule drugs at our disposal, there remains a lack of effective prevention and treatment for many diseases. Fortunately, a new and exciting drug modality has emerged called nucleic acid therapeutics that has the potential to address current unmet needs. This class of drugs uses nucleic acids and closely related compounds for a variety of applications, including regulating gene expression, stimulating the immune system, editing the genome, or supplying new genetic information, with the goal of treating or preventing disease. Thus, learning about nucleic acid therapeutics has important implications for anyone working in health care and related sectors. This advanced course offers a unique way for professionals to learn from leading Harvard Medical School faculty and industry leaders about nucleic acid therapeutics and the advances in this field that have the potential to treat, prevent, and even cure genetic diseases.

Participants will:

- Learn about the various types of nucleic acid therapeutics and their mechanisms of action
- See examples of novel nucleic acid therapeutics that are being used to treat genetic conditions
- Understand the importance of cross-disciplinary collaboration to generate safe and effective nucleic acid therapeutics that can overcome challenges of delivery and toxicity

Topics Covered

Overview of Nucleic Acid Therapeutics

- What are Nucleic Acid Therapeutics?
- The Promise of Nucleic Acid Therapeutics
- Nucleic Acid Immunity and Delivery Challenges

Removing or Modifying Endogenous Information

- Removing Endogenous Information
- ASO Gapmer Therapeutics
- siRNA Therapeutics
- Modifying Endogenous Information
- ASO Steric Blockers for Exon Inclusion
- ASO Steric Blockers for Exon Exclusion
- Clinical Linkage: Treating Duchenne Muscular Dystrophy with ASOs

Supplying New Information

- Introduction to mRNA Medicines
- Interactive: Developing mRNA Medicines
- mRNA Challenges
- In Focus: What is a Vector?

Supplying New Information *cont.*

- mRNA Vaccines
- mRNA Therapeutic Vaccines
- mRNA Therapeutics
- Immunostimulatory Nucleic Acids
- Deep Dive: The Immunostimulatory Effects of mRNA Medicines
- Clinical Linkage: Nucleic Acid-Based Immunotherapy for Melanoma

Permanent Genetic Modifications

- Introduction to Gene Editing
- Gene Editing Considerations
- Gene Editing to Make Deletions
- Templated Modifications
- Base Editing
- Prime Editing
- Clinical Linkage: Treating Sickle Cell Disease with Nucleic Acid Therapeutics

Wrap-up

- The Future of Nucleic Acid Therapeutics

The HMX Pro Series offers a new online learning experience designed to get busy professionals up to speed on the latest advances in medicine. Concepts are taught using whiteboard-style videos and animations and reinforced by interactive elements, true-to life scenarios, and real patient cases to enhance learning.