HMX Short Course - Advances in Genetic Medicines: Approved Therapies in CRISPR, Gene Therapies, and Oligos

Learn about the latest FDA- approved genetic medicines.

While the molecular basis of many genetic diseases has been understood for decades, our ability to treat them has progressed more slowly. Genetic medicines are an emerging class of diverse therapeutics that include small oligonucleotides, gene editing tools, and gene therapies that are capable of altering gene expression or modulating disease-related pathways. With these approaches, we are able to expand the number of druggable targets and treat more conditions including those that lack treatment options.

This advanced course offers a unique way for professionals to learn from Harvard Medical School faculty and industry leaders about recent FDA-approved genetic medicines, including small interfering RNAs, antisense oligonucleotides, gene therapies, and gene editing technologies. You will examine how these therapies are designed and delivered to specific tissues to treat various genetic diseases and the clinical data that led to their approval. By the end of this course, you will be equipped with a strong understanding of relevant genetic medicines and the promise of this exciting field.

Participants will

- Examine how various genetic medicines are delivered to specific tissues—such as the liver, nervous system, eye, skin, and muscle—to treat disease
- Understand how the pathophysiology of each disease shapes the choice of therapeutic approach
- Acquire an in-depth understanding of the current landscape of genetic medicines, including recent advancements and key challenges

Topics Covered Include

- Liver-targeted short interfering RNAs (siRNAs)
- Liver-targeted antisense oligonucleotides (ASOs)
- ASO and Aptamer for Local Delivery
- Deep Dive: Single-Stranded RNA Folding
- Gene Therapies for Sickle Cell Disease, Part 1
- Gene Therapies for Sickle Cell Disease, Part 2
- Gene Therapies for Hemophilia
- Gene Therapies for Neurological Diseases
- Gene Therapies for DMD and Epidermolysis Bullosa
- Expert Conversation: Ongoing Developments in Genetic Medicine

HMX Short Courses feature targeted lessons on the latest medical science information and advancements to keep learners up to date.