HMX Pro Genetics - Gene Therapy

With all the advances in science and with closer collaboration between different scientific disciplines, gene therapy has become a promising treatment option for some genetic conditions and is being heavily investigated for many others. Therefore, learning about gene therapy and how this is carried 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 about gene therapy and about the advances happening in this field that are ultimately helping to improve the treatment of certain genetic diseases.

Participants will:

- Learn the fundamental concepts of gene therapy including the different modalities, critical terminology, and necessary components

- Gain an understanding of the key differences between *ex vivo* and *in vivo* gene therapies, including their unique uses, delivery methods, and challenges

- See how *ex vivo* and *in vivo* gene therapies can be clinically applied to treat inherited genetic conditions

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Topics Covered

Overview of Gene Therapy
- The History of Gene Therapy
- The Promise of Gene Therapy

Introduction to Gene Therapy
- Overview of Gene Therapy
- Gene Therapy Vectors
- Vector Considerations
- Vector Design and Preparation
- Gene Editing
- Gene Therapy Challenges
- Clinical Linkage: Introduction to Inherited Retinal Disorders and Adrenoleukodystrophy

Ex Vivo Gene Therapy
- *Ex Vivo* Gene Therapy Indications
- CAR T Cell Therapy
- Vectors for *Ex Vivo* Gene Therapy
- Conditioning
- Insertional Oncogenesis
- Clinical Linkage: Adrenoleukodystrophy

In Vivo Gene Therapy
- *In Vivo* Gene Therapy Indications
- Vectors for *In Vivo* Gene Therapy
- Delivery of *In Vivo* Gene Therapy
- Overcoming Challenges
- Clinical Linkage: Inherited Retinal Disorders

Wrap-up
- The Future of Gene Therapy

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.

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