

■ Introduction to Gene Therapy

OVERVIEW

Introduction to Gene Therapy provides a foundational understanding of this versatile therapeutic's science and regulatory process. Learn how gene therapy works—the type of vectors used, and why/when certain vectors are employed. This course reviews the most important factors determining which diseases should be targeted using gene therapy. As with any treatment, there are risks, so Introduction to Gene Therapy explains the FDA approval process and how safety and efficacy are measured. Take Introduction to Gene Therapy and gain a basic understanding of gene therapy.

Five Takeaways:

1. Fluency in the different types of viral vectors, the amounts of genetic material each delivers, and the types of cells each targets.
2. Basic principles for transferring genetic material into cells.
3. List the most important factors determining which diseases should be targeted using gene therapy.
4. Learn proven and new approaches to offset gene therapy clinical development risks.
5. Understand the regulatory pathway for gene therapy approval.

AGENDA

Introduction to Gene Therapy

- Genetic basis of human cells
- Gene expression
- Genetic basis of disease
- Causes of monogenic and polygenic diseases
- Process of delivering DNA via gene therapy
- in vivo vs. ex vivo gene therapy
- CAR-T therapy
- Gene therapy administration
- Cell and gene therapy clinical pipeline
- Zolgensma and SMA
- Medical risks and challenges

Viral Vector Delivery Options

- Major viral vector platforms
- Vectors of choice
- AAVs for different cell types
- Targeting non-dividing and dividing cells

- AAV neutralizing antibodies
- AAV vector construct
- AAV9 for SMA
- Manufacturing expression platforms

The Gene Therapy Industry

- Gene therapy landscape
- Key considerations for a gene therapy company
- Ideal gene therapy project traits
- Key IP considerations
- Gene therapy clinical development pathway
- Clinical trial modifications for gene therapy drugs
- Determining efficacy: protein quantification
- Long-term follow-up: durability
- Safety issues: adverse events
- Monitoring adverse events
- Regenerative medicine advanced therapy designation