

**Master Course Recording**  
**Introduction to Gene Therapy**  
Live, Online | Level One

**Introduction to Gene Therapy** Explore how gene therapy works, type of vectors used, and why/when certain vectors are employed. Many believe gene therapy is the key to curing cancer, inherited illnesses, and rare disease; this course reviews the most important factors that determine which diseases should be targeted using gene therapy. As with any treatment, there are risks so this course ends with a look at the FDA approval process and how safety and efficacy are measured. Take Introduction to Gene Therapy and gain a foundational understanding of the science and regulatory environment of this versatile therapeutic.

**Five Takeaways**

1. Basic principles for transferring genetic material into cells and using viral vectors.
2. Steps to make viral vectors.
3. Fluency in the different types of viral vectors, the amounts of genetic material each deliver, and the types of cells each target.
4. List of the most important factors that determine which diseases should be targeted using gene therapy.
5. Proven and new approaches to mitigate risk in gene therapy clinical development.

**Week One**

**Gene Therapy Overview** *60 minutes*

- Basic cell biology: DNA to RNA to Protein
- Genetic disorders: DNA's role in disease
- Gene therapy: in vivo and ex vivo
- Therapeutic areas: hematological, ophthalmic, musculoskeletal, neurological, metabolic, hepatological, oncological, infectious
- How gene therapy works
- SMA/Zolgensma example
- Choice of viral vector and why
- Packaging size
- Tissue tropisms
- Safety and immunogenicity
- Dosage

**Week Two**

**Viral Vector Overview** *40-minutes*

- Gene transfer
- Tissue specificity
- Delivery methods
- Vectors: AAV, adino virus, lentivirus, retrovirus, vaccinia and MV

**Week Three**

**Pharma Industry & Gene Therapy** *45-minutes*

- FDA regulatory overview
- Measuring safety and efficacy
- Zolgensma approval: What metrics were analyzed
- Gene therapy drug development process
- Novel disease targets
- Survey of gene therapy companies

**Course Assessment** *20 minutes*

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