

Targeted Biologics: Introduction to Cell and Gene Therapies

For those who already have a basic understanding of biology

Targeted Biologics: Introduction to Cell and Gene Therapies is an accelerated, one-day course focused on the most innovative drugs currently in development or newly on the market. These therapeutics take their inspiration from the workings of our own immune system, so the morning begins with the basics of immunology. The rest of the day delves into the science, development challenges and promise of cell and gene therapies. If you have a firm grasp of basic biology, this course will take your understanding of the healthcare industry to the next level.

Five Takeaways

1. The rationale behind cancer immunotherapies
2. The challenges and second-generation opportunities for immunotherapies
3. Differentiation between the types of DNA- and RNA- based therapies
4. Improved understanding of gene therapy and genome editing
5. Ability to discuss multiple applications of genome editing

Agenda

Immunology Overview 9:00-10:00

Activation of the immune system
B-cells
Antibodies: structure and function
Memory B-cells
T-Cells
Cytotoxic T-cell structure and function
Helper T-cell structure and function
Regulation of the immune system
Cytokines, PD-1 and CLTA-4

Break 10:15-10:30

CAR-T Overview 10:30-11:15

How cells of the immune system are used for cell therapy
T-cell biology
Introduction to CAR-T therapy
CAR-T indications: blood cancers, solid tumors
CAR-T principles:
What is a CAR-T
How are they made?
CAR-T: Off-the-shelf and patient-specific

CAR-T Overview *continued* 10:30-11:15

CAR-T safety: controlling activation
CAR variations: CAR-NK, CAR-MA, TCR therapies, bispecific CAR

Gene Therapy: The Big Picture 11:15-12:00

What is gene expression?
Gene therapy modalities: viral vector, gene editing, antisense, mRNA, RNAi,
Gene therapy another way: in vivo and ex vivo
Therapeutic areas: hematological, ophthalmic, musculoskeletal, neurological, metabolic, hepatological, oncological, infectious
How does the FDA regulate targeted biologics?

Lunch 12:00-12:45

Viral Vectors Overview 12:45-1:45

DNA's role in disease
Monogenic and polygenic disease
How gene therapy works: targeted and systemic
Gene transfer

Viral Vectors Overview *continued* 12:45-1:45

Delivery methods

- Vectors: AAV, lentivirus, others

- Choice of viral vector and why

- Packaging size

Tissue tropisms

Safety: immunogenicity and dosage

Approved and clinical viral gene therapies

Opportunities and risks

Genome Editing Overview 1:45-2:45

Zinc finger nucleases (ZFN)

- ZFN therapeutic areas

- How ZFN work

- ZFN in the clinic

- ZFN Safety

CRISPR

- CRISPR therapeutic areas

- How CRISPR works

- CRISPR Safety

- CRISPR in the clinic

 - PD1 Knockouts

 - Inherited retinal dystrophy, beta-thalassemia

Exon skipping

- Exon skipping therapeutic areas

- How exon skipping works

- Exon skipping in the clinic

- Exon skipping safety

Break 2:45-3:00

RNA-Based Therapeutics Overview 3:00-4:15

RNA's role in the cell

RNA's role in disease

Therapeutic areas

Types of RNA-based therapeutics

- Antisense

- RNAi

- mRNA (exon-skipping)

How RNA-based therapeutics work

Safety

RNA-based therapeutics approved/in the clinic

Wrap-Up 4:15-4:30