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

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

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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

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