

# Novel Drugs: Immuno, Gene and Cell Therapies Course Agenda

**Novel Drugs: Immuno, Gene and Cell Therapies** course focuses on the most innovative drugs currently in development or newly on the market. The inspiration for these emerging medicines is our own immune system, so we begin with a look at immunology. The rest of the day is spent learning about the science, development challenges and healthcare potential of immunotherapies, gene therapies and cell therapies.

## 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

## Course Agenda

### **Immunology: How the Body Fights**

**Disease 9:00-10:15**

Immune system cells and tissues  
Non-specific response: inflammation  
Cytokines  
Autoimmune and inflammatory disease  
Inflammation and obesity  
Specific response: T-cells and B-cells  
Cytotoxic T-cell structure and function  
Helper T-cell structure and function  
Regulatory T-cell structure and function  
Immune system activation

**Break 10:15-10:30**

### **Immunotherapies Part 1 10:30-12:00**

Tumor microenvironment  
How tumor cells evade the immune system  
Checkpoint inhibitor therapies  
Next generation checkpoint inhibitors  
Chimeric antigen receptor therapies (CAR-T)  
CAR variations  
CAR-T therapy for blood cancers

Preparation and expansion of CD19-CAR-T lymphocytes  
CAR-T for solid tumors

**Lunch 12:00-1:00**

### **Immunotherapies Part 2**

CAR-T challenges  
Process-specific  
Safety  
CAR-T 2.0: controlling activation  
Bispecific CAR-T  
On/off switches  
Oxygen-sensitive CAR-T  
Ultrasound activation  
Off the shelf CAR-T  
CAR-T in autoimmunity, HIV  
CAR-NK, CAR-MA, TCR Therapies

### **DNA- and RNA-Based Therapeutics**

**1:45-3:00**

DNA role in disease

RNA therapeutics: Antisense, siRNA,  
microRNA, mRNA

Gene transfer

Ex vivo vs. in vivo gene delivery

Gene therapy

Viral vectors used in gene therapy

Genome editing: zinc finger nuclease

Gene transfer vs. genome editing

Genome editing: curing HIV?

Genome editing: CRISPR/CAS9

**Break 3:00-3:15**

**CRISPR Applications 3:15-4:15**

CRISPR in the clinic

CRISPR in agbiotech

RNA editing

CRISPR-based diagnostics

Base-editors

CRISPR as an antibiotic

**Q&A | Evaluation 4:15-4:30**