

BioBasics 201: Targeted Biologics for the Non-Scientist

Vaccines, Cell, Gene, Antibody, and RNA Therapies

Live, Online | Level Two Suggested prerequisite BioBasics 101

BioBasics 201: Targeted Biologics for the Non-Scientist is an intensive, two-day course focused on vaccines, cell therapies, gene therapies, therapeutic antibodies, and RNA-based drugs. Biopharma's inspiration for the development of targeted biologics is our own human immune system, so we begin with an in-depth explanation of immunology. The course then focuses on the science, challenges, and medical promise of next generation targeted biologics. BioBasics 201 is designed for the non-scientist who has taken BioBasics 101 or for those who understand the basics of DNA, RNA, proteins, and cell signaling. Taught by industry experts who explain how these complex drugs work in a simple, engaging manner.

Five Takeaways

1. Understand the mechanisms used by the human body to protect itself against disease.
2. Fluency in vaccine platforms, how each protects against disease including Covid-19.
3. Rational of therapeutic antibody mechanisms of action, including monoclonal antibodies, antibody-drug conjugates, bispecific antibodies, checkpoint inhibitors.
4. Compare and contrast types of DNA- and RNA-based therapies and how each cures disease.
5. Increased knowledge of cell therapies, including all variations of CAR-based therapies.

Agenda

Day One

Immunology: Intro to the Human Immune System 9:00-10:00

Tissues of the immune system
Non-specific and specific immunity
Key immune cell roles
Immune signaling: cytokines
Industry application: cytokine storm

Break 10:00-10:15

Immunology: How Our Body Fights Disease 10:15-11:30

Non-specific immune response
Industry application: inflammation
Specific immune response
Activation of the immune system
B-cells
Antibodies: structure and function
Industry application: monoclonal antibodies

Immunology: How Our Body Fights

Disease *continued*

Complement response

T-cells

Regulation of the immune system

PD-1 and CTLA-4

Industry application: tumor suppression of T-cells

Break 11:30-11:45

Immunotherapies: An Overview 11:45-12:15

Immunotherapy defined

Immunotherapies review

Therapeutic antibodies

Oncolytic virus therapy

Vaccines

Cell therapies (CAR-T)

Lunch 12:15-1:00

Targeted Biologics: Vaccines 1:00-1:30

Immunological memory

How vaccines work

Vaccine platforms

DNA and RNA vaccines

Industry application: universal flu vaccine

Focus On: Covid-19 1:30-2:15

Morphology and virus activity

Covid-19 vaccines

How does an RNA vaccine work?

Antivirals and therapeutic antibody drugs

Break 2:15-2:30

Focus On: Oncology 2:30-3:15

Cancer

Growth factor signaling

Industry application: Gleevec

Immunosuppressive tumor microenvironment

Cancer immunotherapy

Wrap-Up 3:15-3:30

Day Two

Targeted Biologics: Therapeutic Antibodies

9:00-10:00

Therapeutic antibodies

Industry application: polyclonal vs monoclonal antibodies

Therapeutic antibody mechanisms of action

Antibody-drug conjugates

Bispecific antibodies

Checkpoint inhibitors

Industry application: PD-1 and PD-L1

Industry application: CTLA-4

Next generation checkpoint inhibitors

Break 10:00-10:15

Targeted Biologics: Cell Therapies 10:15-11:15

How immune cells are used for cell therapy

CAR structure and function

Selected CAR therapies

CAR variations: CAR-NK, CAR-MA TCR

Industry application: targeting solid tumors

Autologous vs allogeneic cell therapies

How are CARs made?

CAR-T safety: controlling activation

Industry application: CAR treatment for autoimmunity

Break 11:15-11:30

Targeted Biologics: RNA Therapies

11:30-12:30

RNA's role in the cell

RNA's role in disease

Therapeutic areas

Types of RNA-based therapeutics

Antisense

Industry application: Kynamro

Exon-inclusion and exon-skipping

Industry application: Spinraza

siRNA therapies

Lunch 12:30-1:15

Targeted Biologics: Gene Therapies 1:15-2:15

Gene therapy: in vivo and ex vivo
DNA deliver via viral vectors
Viral vector platforms
Gene therapy composition
AAV and lentivirus characteristics
Industry application: Luxtuma
Industry application: Zolgensma
AAV neutralizing antibodies
Gene therapy and biomarkers
Durability of effect
RMAT designation
Risks and challenges

Targeted Biologics: Genome Editing 2:15-3:15

Gene therapy vs genome editing
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
 Industry application: PD-1 knockouts
 CRISPR Babies activity
 CRISPR as RNA editor
 CRISPR diagnostics
 Industry application: SHERLOCK and
 DETECTR

Wrap-Up 3:15-3:30