



# **BioBasics 201 Targeted Biologics for the Non-Scientist**

Vaccines, Cell, Gene, Antibody, and RNA Therapies

LIVE, ONLINE | LEVEL TWO | SUGGESTED PREREQUISITE BIOBASICS 101

# **OVERVIEW**

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

Complement response

T-cells

Regulation of the immune system

PD-1 and CLTA-4

Industry application: tumor suppression of

T-cells

Break 11:30-11:45

**Immunotherapies: An Overview** 11:45-12:15

immuotherapy defined

Immunotherapies review

Therapeutic antibodies

Oncolytic virus therapy

Vaccines

Cell therapy (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-Based 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 Therapy** 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: Zolgensa

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