# Basic Drug Development: Overview of Biopharmaceutical R&D

*August 7 - 8, 2023 • Boston, MA*

***Program Day One: Monday, August 7, 2023*** ***Session No.***
*All times listed EDT*

8:15 AM ***Classroom Opens, Continental Breakfast***

8:30 – 8:45 AM **PERI Welcome and Introductions**

- Participant introductions
- Faculty Introductions
- Course Goals

8:45 – 9:45 AM **Overview of Pharmaceutical Development, Metrics and Top Issues** ***1***
Allan J. Weinstein, MD

- Overview of Development Process - From Candidate to Market
- Drug Development Metrics
- Key Development Questions – Go/No Go Decision Points
- Current Industry Trends & Future Developments
- Pharmaceutical Spending: Global Development
- Success Rates in Development
- Patient-Reported Outcomes

9:45 – 10:30 AM **Begin with the End in Mind: Label Driven Drug Development** ***2***
Allan J. Weinstein, MD

- What is in a submission for market authorization?
- Target Product profile (TPP)
  - Have a structured way to develop a target product profile
  - Looking at competitors, market landscape
- Commercial involvement

10:30 – 10:45 AM ***Stretch Break #1***

10:45 AM – 12:00 PM **Drug Discovery** ***3***
Noel J. Cusack, PhD

- Targets for drug intervention
- Sources of new drug candidates
- Optimization of drug candidates
- Drug candidate validation
- Drug discovery exercise

| ***Program Day One, continued***<br>*All times listed EDT* | | ***Session No.*** |
|---|---|---|
| 12:00 – 12:45 PM | ***Catered Lunch Break*** | |
| 12:45 – 1:45 PM | **Pharmacokinetics: Issues and Considerations**<br>Noel J. Cusack, PhD<br>• Refresher: Key Concepts in PK & ADME<br>• PK support during drug development<br>• Comparison of biologics with small molecules<br>• Case studies | ***4*** |
| 1:45 – 2:45 PM | **Portfolio Management Workshop 1: "Early Development Decision Making"**<br>Faculty<br>• Participants assume the role of a "Portfolio Planning Committee"<br>• The assignment: select 4 candidates to advance based on the pre-clinical information provided for a list of 10 compounds/therapies<br>• Group discussion of findings and decision-making rationale | ***5*** |
| 2:45 – 3:00 PM | ***Afternoon Break*** | |
| 3:00 – 4:00 PM | **Key Concepts in Nonclinical Safety and Toxicology**<br>Noel J Cusack, PhD<br>• Objectives of Nonclinical Safety for Clinical Trial Planning<br>• Nonclinical safety assessment (Risk)<br>• Dose selection for First-in-Human clinical trial<br>• Safety support during drug development<br>• Case studies | ***6*** |
| 4:00 – 4:05 PM | ***Stretch Break*** | |
| 4:05 – 4:50 PM | **Chemistry, Manufacturing and Controls (CMC)**<br>Noel J. Cusack, PhD<br>• Drug Substance manufacture<br>  ○ Small molecules<br>  ○ Biologics<br>• Drug Product manufacture<br>  ○ Rationale for formulation<br>  ○ Small molecules<br>  ○ Biologics<br>• Case studies | ***7*** |
| 4:50 – 5:00 PM | **Course Day 1 Wrap-up, Preview Day 2** | |

***Program Day Two: Tuesday, August 8, 2023*** **Session No.**
*All times listed EDT*

8:15 AM ***Classroom Opens, Continental Breakfast***

8:30 – 8:35 AM **PERI Welcome: Day Two Preview**

8:35 – 9:45 AM **Regulatory Overview** ***8***
Allan J. Weinstein, MD

- Regulations for new drug and biologic approval
  - Overview of regulatory process
  - Understanding the regulatory agencies' role in clinical development: US, EU, Japan
  - Regulatory Update: changes in last 10 years
- Emerging Regulatory Landscape

9:45 – 9:50 AM ***Stretch Break #1***

9:50 – 10:50 AM **Portfolio Management Workshop 2: "Advancing Compounds"** ***9***
Faculty

- "Portfolio Planning Committee" assignment (continued)
- Select 2 compounds to advance based upon recently generated pre-clinical information provided
- Decide the fate of all 10 candidates
  - Develop, out-license, put on shelf, or kill
- Group discussion of findings and decision-making rationale

10:50 – 11:00 AM ***Stretch Break #2***

11:00 AM – 12:15 PM **Clinical Development Plan Considerations – Part 1** ***10***
Allan J. Weinstein, MD

- Goals of a clinical development program
- Expectations of regulatory agencies
- Phases and expectations of clinical research
- Strengths and weaknesses of clinical Trials
- Adaptive design
- Bayesian statistics
- Big Data

***Program Day Two, continued*** **Session No.**
*All times listed EDT*

12:15 – 1:00 PM ***Catered Lunch Break***

1:00 – 2:15 PM **Clinical Development Plan Considerations – Part 2** ***11***
Allan J. Weinstein, MD

- Study designs
- Challenges of global development
- The protocol
- Epidemiology of disease
- Good clinical practices
- Ethical considerations

2:15 – 3:15 PM **Portfolio Management Workshop 3 – "Selecting Development Strategy"** ***12***
Faculty

- Limited clinical trials conducted on 4 of the drug candidates
- Only two candidates considered for further development
- Nonclinical safety and CMC data obtained for the 2 candidates
- What could a sales force use as a "handle" for eventual marketing?

3:15 – 3:30 PM ***Afternoon Break***

3:30 – 4:15 PM **Pharmacoeconomics and Market Access** ***13***
Allan J. Weinstein, MD

- Pharmacoeconomics & Healthcare Outcomes Research
- Measuring the value of pharmaceutical therapy
- Healthcare Informatics: QoL, QALY
- Pricing & payer reimbursement issues
- Cost-minimization, benefit, utility, comparative effectiveness
- PROs: Patient Reported Outcomes
- Value evidence data generation

4:15 – 4:45 PM **Product Launch** ***14***
Allan J. Weinstein, MD

- Pre-launch necessities: regulatory and business requirements
- Developing the launch plan
  - Launch team, distribution strategy, metrics for success
  - Product lifecycle

4:45 PM **Course Conclusion**

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## Course Overview

PERI's fundamental education course provides an overview of the pharmaceutical development process, from drug discovery, nonclinical and clinical development, and manufacturing, culminating in product launch and marketing. The course focuses on the decisions that need to be made throughout the therapeutic development process and the criteria influencing these decisions. In addition to face-to-face training, the Decision Points in Pharmaceutical Development online component introduces the phases of development and the key concepts explored during the live course.

## Course Objectives

*Upon completion of this course, participants should be able to:*

- State the basic steps in the drug development process
- Explain the difference between research and development
- Analyze the key decision points in the drug development process
- Discuss the role of interaction with FDA and other regulatory agencies

## Course Faculty

*Noel J. Cusack, PhD*
Independent Non-Clinical Consultant
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*Allan J. Weinstein, MD*
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## Continuing Education Credit

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