NDA Simulation Workshop: Pharmaceutical Development Decisions
April 24 – 25, 2023
4000 Legato Road, Suite 1100, Fairfax, VA

Monday, April 24, 2023
All Times Listed EDT

Session No.

8:00 – 8:30 AM **Registration & Continental Breakfast**

8:30 – 8:45 AM **PERI Welcome & Course Introduction**

- Course overview
- Faculty introductions
- Participant introductions

8:45 – 10:00 AM **Regulatory Overview** — **1**
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 (PDUFA VII)

10:00 – 10:15 AM ***Morning Break***

10:15 – 11:15 AM **Chemistry, Manufacturing and Controls** — **2**
Noel J. Cusack, PhD
Independent Consultant, Nonclinical Development

- Drug Substance (Active Pharmaceutical Ingredient)
  - Small molecules
- Drug Product (Formulated Drug Substance)
  - Small molecules
- Rationale for formulation
  Case studies

11:15 AM – 12:30 PM **Key Concepts in Nonclinical Safety and Toxicology** — **3**
Noel J. Cusack, PhD

- Objectives of Nonclinical Safety for Clinical Trial Planning
  - Small molecules
- Nonclinical safety assessment (Risk)
- Dose selection for First-in-Human clinical trial
- Safety support during drug development
- Case studies

12:30 – 1:15 PM ***Lunch Break***

1:15 – 2:00 PM	**The IND Document: Incorporating Preclinical Data** Noel J. Cusack, PhD • Structure of an Investigational New Drug (IND) Application • Incorporating Preclinical Data	**4**
2:00 – 3:15 PM	**The IND Simulation Workshop** **Part 1: Introduction & Submission of IND** Participants and Faculty • Workshop Introduction • Group Work & Faculty Consultations	**5**
3:15 – 3:30 PM	***Afternoon break***	
3:30 – 4:45 PM	**The IND Simulation Workshop** **Part 2: Group Presentations** Participants and Faculty • Group Presentations • Lessons Learned	**6**
4:45 – 5:00 PM	**Course Day 1 Wrap-up**	

Tuesday, March 25, 2023
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Session No.

8:00 – 8:30 AM **Continental Breakfast**

8:30 – 9:30 AM **Clinical Development Plan Considerations – Part 1** — **7**
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

9:30 – 10:30 AM **Clinical Development Plan Considerations – Part 2** — **8**
Allan J. Weinstein, MD

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

10:30 – 10:45 AM ***Morning Break***

10:45 AM – 11:45 AM **The NDA Document: Incorporating Clinical Data** — **9**
Allan J. Weinstein, MD

- Structure and format of a New Drug Application (NDA)
- Incorporating Clinical Data

11:45 AM – 1:00 PM **The NDA Simulation Workshop** — **10**
Part 1: Introduction to NDA Simulation
Participants and Faculty

- Team building and role playing
- FDA as interactive participants
- Decision-making in the drug development process
- The evolving product label

1:00 – 2:00 PM ***Lunch break***

2:00 – 3:30 PM **The NDA Simulation Workshop** — **11**
Part 2: NDA Assembly and FDA Interactions
Participants and Faculty

- The evolving product label
- Consultant interactions

3:30 – 3:45 PM ***Refreshment Break***

3:45 – 4:45 PM	**The NDA Simulation Workshop** **Part 3: Label Submission to "FDA"** Participants and Faculty • Sponsor company NDA presentations to "FDA" panel • Final product label • Approval of indication • Brand NAME • Lessons Learned	**12**
4:45 – 5:00 PM	**Course wrap-up and adjournment**	