Basic Drug Development:
Overview of Biopharmaceutical Research & Development

Courtyard by Marriot, Vienna, VA
May 16 – 17, 2019

Thursday, May 16, 2019 ***Tab No.***

7:30 – 8:00 AM ***Registration***

8:00 – 8:15 AM **PERI Welcome**
Jo Ann Zoul
Course Manager

Introductions and Expectations
Faculty and Participants

- What knowledge bases do we need?
- Group's current understanding of the major phases and decision-points regarding how new products are identified, evaluated, developed, reviewed, regulated and marketed
- Participants' introductions and brief descriptions of their roles

8:15 – 9:00 AM **Overview of the Biopharmaceutical Research & Development Process** ***1***
Noel J. Cusack, PhD *Course Director*
Independent Consultant
Nonclinical Development

- An overview of the major decision points/phases of the biopharmaceutical development and approval processes.

9:00 – 10:00 AM **Overview of Nonclinical Biopharmaceutical Development** ***2***
Noel J. Cusack, PhD

- Safety and toxicity assessment (risk)
- Pharmacokinetics (drug handling)
- Dose selection for First in Human clinical trial
- Nonclinical studies to support drug NDA/BLA and marketing approval

10:00 – 10:15 AM ***Morning Break***

10:15 AM – 12:15 PM **What Influences and Determines How Clinical Trials are Designed** ***3***
M. Scott Harris, MD
Principal, Middleburg Consultants

- Defining the clinical outcomes and corresponding clinical endpoints
- Developing a clinical protocol--how to avoid multiple amendments
- Addressing the key elements of a clinical protocol
- Analyzing the endpoints of clinical trials
- Developing a clinical protocol in US, EU and Japan

12:15 – 1:15 PM ***Lunch on your Own***

1:15 – 2:15 PM **A Review of Safety in Drug Development** **4**
M. Scott Harris, MD
Principal, Middleburg Consultants

- Sources of safety data: pre-approval and post-approval
- Pharmacovigilance Practices, RMPs, and REMS
- FDA communication initiatives

2:15 – 3:15 PM **Workshop 1 – Selecting Development Candidates** **5**
Faculty and Participants

- Participants assume the role of a "Portfolio Planning Committee"
- The assignment: select 4 drug candidates to advance based on the preclinical information provided for a list of 10 compounds/therapies
- Group presentation of findings and decision-making rationale
- Reassess selection of drug candidates based on new information

3:15 – 3:30 PM ***Afternoon Break***

3:30 – 4:30 PM **Chemistry, Manufacturing and Controls (CMC)** ***6***
Noel J. Cusack, PhD

- Drug Substance manufacture
  - Small molecules
  - Biologics
- Drug Product manufacture
  - Rationale for formulation
  - Small molecules
  - Biologics

Friday, May 17, 2019 ***Tab No.***

8:00 – 9:15 AM **Regulatory Overview** ***7***
Allan J. Weinstein, MD
Clinical Research and Regulatory Affairs Consultant

- Regulations for new drug 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 VI)

9:15 – 10:30 AM **Workshop 2 – "Assigning Clinical Candidates"** ***8***
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?

10:30 – 11:00 AM ***Morning Break***

11:00 AM – 12:30 PM **FDA Presentations & Panel Discussion** ***9***
Amy Ellis, PhD
Pharmacologist
Division of Anti-Infective Products
CDER/OND/OAP

Kamal N. Tiwari, PhD
Quality Assessment Lead (Acting)
Division of Process Assessment III, Branch IX
CDER/OPQ/OPF

12:30 – 1:30 PM ***Lunch on Your Own***

1:30 – 2:45 PM **Workshop 3 – "Selecting Drug Development Strategy"** ***10***
Faculty

2:45 – 3:00 PM ***Afternoon Break***

3:00 – 4:00 PM **Pharmacoeconomics and Market Access** **11**
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
- Reimbursement linked to outcomes
- PROs: Patient Reported Outcomes
- Value evidence data generation

4:00 – 4:15 PM **Program Wrap-up**
Faculty

- Question and Answer session
- Recap of the goals of the program and content covered

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