BIOTECH PRIMER

COURSE SNAPSHOT

Drug Development

biotechPrimer
Life Science Training From Industry Experts

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ABOUT US

Biotech Primer Inc. develops and delivers training to help professionals understand the science, business, and regulatory processes essential to the biotechnology, pharmaceutical, molecular diagnostics and medical device industries. Our industry experts continuously create, update and deliver the most engaging instruction anywhere. We have the experience and expertise needed to prepare companies to make strategic business decisions, navigate important regulatory hurdles, and move healthcare products from the bench to the bedside. To accomplish these goals, we offer a diverse range of learning opportunities, ensuring participants retain and put into practice what they learn.

- Integrate your science and business operations
- Bring in-depth knowledge to your sales force
- Help your team converse more effectively with industry clients, colleagues, and scientists
- Enable your entire staff to recognize new opportunities

OUR SUBJECT EXPERTISE

- Biotechnology for Non-Scientists
- Drug Development
- Drug Manufacturing
- Business of Biotech
- Molecular Diagnostics
- Medical Devices

OUR DELIVERY PLATFORMS

Our training is offered using multiple platforms to better fit your learning preferences and scheduling constraints

- **Live Customized Courses** Tailored training delivered to organizations worldwide live online or live onsite. You can modify the master course agendas to meet your specific learning needs.
- **Live Master Courses** Prescheduled courses for individuals or schedule a master course for your organization.
- **Recorded Master Courses** Offers the same content, exercises and workbook as the live master course, with the ability to take the course on-demand, online when your schedule permits.

- **On-Demand, Short Classes** Short interactive classes for individuals or bulk purchased for organizations.
  - Class transcripts and subtitles available in 9 languages including English, Japanese, Chinese, Spanish, French, French Canadian, Hindi, Arabic, and Russian.
  - Certificate available upon successful class completion.
  - Ability to upload certificate to your LinkedIn education profile.
  - Two corporate account options available for on-demand, short classes
    - **Enterprise:** Manage your own company account with our Learning Management System (LMS). Assign classes and view individual's progress. Enterprise is intuitive and easy-to-manage. No extra cost.
    - **LTI Bridge:** Connect your organizations LMS to Biotech Primer's LMS. Participants log into their company's LMS and take our classes. Integration costs apply.

OUR PRICING

- **Live Customized Courses** The cost of tailored training depends on content, length of course, and number of participants. Discounts given to multiple classes purchased within one year.
- **Live Master Courses** Prescheduled two-day courses for individuals range from $1495-$1695 US.
- **Recorded Master Courses** These courses are 3-12 hours in length and cost $895 US. Participants are given three months to complete each course.
- **On-Demand, Short Classes** Each class is $150 US. BIO members receive special pricing of $120 US per class. Participants are given two weeks to complete the class.

Bulk discount pricing:

Number of total classes	Discount per class	Price per class
10-20*	25%	$112
21–100	30%	$105
101–250	40%	$90
251–500	50%	$75
500 and up	70%	$45

**For BIO member companies only*

OUR COURSE LEVELS

- **Level 1: Foundational** For non-scientists new to biopharma and for those who need a refresher on the fundamental science driving the healthcare industry.
- **Level 2: General** For individuals who possess a general understanding of science basics.
- **Level 3: Advanced** For individuals who have a good grasp of the science.

OUR PUBLICATIONS

- **The Biotech Primer One: The Science Driving Biopharma Explained**
  Learn the basic science driving the biopharma industry in this fully illustrated 120-page book.
- **The Biotech Primer Two: Next Generation Therapies Explained**
  Learn how vaccines, therapeutic antibodies, cell therapy, gene therapy, and RNA therapeutics mitigate disease in this easy-to-read 170-page book. Books available for purchase on Amazon.
- **The WEEKLY**
  White papers that explain the science behind the headlines.

OUR INSTRUCTORS

Biotech Primer instructors offer extensive industry experience. By drawing on their various backgrounds, these seasoned professionals are well-informed on the real-world situations you face. They have developed drugs, diagnostics, and medical devices for companies ranging from multinational corporations to start-ups.

Biotech Primer courses are on point, thorough and taught by one dedicated industry educator, not a patchwork of invited academic lecturers.

You can expect:

- Limited class size so all your questions are answered
- Hands-on labs, thought-provoking case studies, and dynamic discussions so you practice what you learn
- Industry war stories to help you avoid lessons hard learned by others

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LIVE MASTER COURSE | LEVEL ONE
SUGGESTED PREREQUISITE: NONE

Drug Discovery for the Scientist

OVERVIEW

Drug Discovery for the Scientist is a one-day, intensive course specifically designed for scientists who need to understand the drug discovery process better. In this course, you will learn how to apply a risk assessment and use multi-disciplinary approaches with considerations for how best to get medicine to patients. Learn from an industry expert what it takes to validate targets and screen molecules to select a lead candidate to inform an investigational new drug (IND) or clinical trial application (CTA).

Five Takeaways:

1. Fluency in drug discovery process including major milestones.
2. Selection of pharmacologically relevant targets.
3. Criteria to eliminate candidates.
4. Key development considerations for antibodies, cell and gene therapies.
5. Required regulatory studies and data to enable an IND or CTA.

AGENDA

Target Validation 45 minutes
Drug development process overview
Stage gates
Survey of druggable and undruggable targets
Target ID, selection, key considerations
Target platforms and libraries

Break 15 minutes

Lead Optimization 75 minutes
How to prioritize leads
High-throughput screening techniques
How to eliminate candidates
Affinity maturation, antibody diversity
IgG subtype, alternate formats

Packet Insert Activity 15 minutes

Cell-Based Therapy 30 minutes
Screening systems: in silico, in vivo, in vitro
Identifying potential safety issues

Lunch 45 minutes

Safety: Off-Target Screening 30 minutes
Screening systems: in silico, in vivo, in vitro
Identifying potential safety issues

Break 15 minutes

Analytical and Bioanalytical Assays 30 minutes
Assay development
Assay validation
Supporting cGMPs and GLP

Enabling Clinical Trials 45 minutes
Quality
Pharmacology
Pharmacokinetics
Toxicology
Required studies/data to enable IND/CTA

Wrap-Up 15 minutes

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LIVE MASTER COURSE | LEVEL ONE
SUGGESTED PREREQUISITE: NONE

Preclinical Development for the Scientist

OVERVIEW

Preclinical Development for the Scientist is a one-day, fast-paced course specifically designed for scientists who need to better understand the regulatory requirements of preclinical development. Learn from an industry expert the approaches and criteria used to support a development candidate's nomination for an IND or CTA to enable first-in-human studies.

Five Takeaways:

1. Fluency in preclinical/nonclinical development including required milestones.
2. Typical approaches and criteria used to support development candidate nominations for first-in-human trials.
3. Species selection and use of animal pharmacokinetics in describing the expose-response relationships.
4. Importance of toxicology in selecting compounds and establishing safety.
5. Estimating safe starting dose levels for clinical trials.

AGENDA

CMC 45 minutes
Upstream and downstream bulk processing
Cell line and cell bank development
Formulation, fill, and finish
Stability and analytical testing of protein products

Pharmacology 60 minutes
Required data including publications
Binding, potency, receptor occupancy
Efficacy improvement
Qualitative and quantitative endpoints
In vitro and in vivo models

Break 15 minutes

Pharmacokinetics 30 minutes
Absorption, distribution, metabolism, elimination
PK/PD, exposure-response relationships
Noncompartmental analysis, AUC, Cmax

Toxicology 45 minutes
Criteria to support candidate nomination
Dose level selection, routes of administration
Species selection, therapeutic margins
Defining adverse effects
Estimating safe starting dose levels

Lunch 45 minutes

Non-Clinical Development Considerations in Pre-IND/IND 45 minutes
Quality
Pharmacology, pharmacokinetics, and toxicology
Required regulatory studies/data to enable IND/CTA

Break 15 minutes

Integrated Development 45 minutes
Packaging CMC/nonclinical/clinical data for IND

IND Requirements 30 minutes
IND components
Common technical document format
Regulatory process

Wrap-Up 15 minutes

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LIVE MASTER COURSE | LEVEL ONE
SUGGESTED PREREQUISITE: NONE

Drug Development Immersion

OVERVIEW

Drug Development Immersion is a two-day, interactive course that explores the regulatory, commercial, and scientific factors needed to bring a drug successfully to market. The discussion features both small molecule drugs and biologics. Our instructors illustrate the corporate decision-making process with personal accounts, giving participants unique strategic development insights. Learn from an industry expert what it takes to get a molecule from the bench into the marketplace.

Drug Development Immersion was developed for the non-science professional who works within or services the biopharma industry and needs to become familiar with the preclinical and clinical development process.

Five Takeaways:

1. Fluency in essential terminology and acronyms used in clinical development.
2. An in-depth look at the FDA and EMA regulatory process and sponsor interactions.
3. Criteria for preclinical studies to support first-in-human clinical trials.
4. Rationale, special considerations, and study design for both traditional and non-traditional clinical trial phases.
5. Understanding of the launch process, lifecycle management, and post-approval drug safety monitoring.

AGENDA

DAY ONE

Introductions 15 minutes

Setting the Stage 75 minutes
Small and large molecule drug characteristics
Desirable drug characteristics
Agonist and antagonist drugs
Route of administration based on drug type
Traditional drug development pathway
Gene and cell therapy development pathway
Drug development metrics
Chances of success, timelines, and costs

Break 15 minutes

The Business of Drug Development 75 minutes
Integrated drug development process
Stage gates: go/no go decisions
Target product profile
Draft label
Activity: Understanding the Draft Label
US patents and market exclusivity

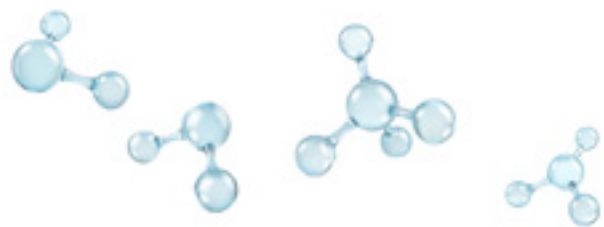
Lunch 45 minutes

The Regulatory Process 75 minutes
Regulatory agencies and compliance worldwide
PDUFA, GDUFA, BsUFA
Generics and biosimilars approval pathways
FDA/sponsor meeting timeline
FDA expedited programs
Voucher system
FDA and EMA orphan drug designation
EMA user fees and review times
EMA expedited reviews and designations
FDA and EMA approval process
Regulatory compliance

Break 15 minutes

Preclinical Development 60 minutes
Preclinical development pre-IND/CTA
Preclinical data objectives
Safety testing terms
Nonclinical studies
Toxicology, pharmacology, pharmacokinetics
IND/CTA filings
Authorization to proceed to clinical trials

Wrap-Up 15 minutes



DAY TWO

The Players: Who is involved? 45 minutes
Subjects, sponsors, investigators
Ethics committees/investigational review board
Informed consent
Contract research organizations
Patient advocacy groups
Data monitoring committees (DMC)
How DMC impacts clinical trials

General Principles: Ethics and Risk 45 minutes
Risk assessment and management
Bias and data integrity
Controlling bias: blinding and randomization

Break 15 minutes

Conduct of Clinical Trials 60 minutes
Clinical research purpose
Introduction to study design elements
Endpoints
Inclusion/exclusion criteria
Placebos and control groups
Adverse events and safety reports
Clinical trial documentation
Data management and trial master files

Clinical Development Phase I 45 minutes
Purpose of Phase I
Design and conduct of Phase I
Selection of dose: MAD and SAD
Phase IA and IB
Bioequivalence trials
Combined Phase I/II studies
Combining Phase I/II trials

Lunch 45 minutes

Clinical Development Phase II 45 minutes
Purpose of Phase II
Phase IIA and IIB
Randomized control trials
Statistical considerations
Null hypothesis, P value, type 1 and 2 errors
Activity: Introduction to Clinical Statistics

Clinical Development Phase III 45 minutes
Purpose of Phase III
Phase IIIB
Trial designs: parallel, crossover, adaptive
Database cleaning, lock and unblinding
Regulatory application submittal

Clinical Development Phase IV 30 minutes
Real-world evidence initiatives
Launch and lifecycle management
Drug safety and pharmacovigilance

Wrap-Up 15 minutes

Drug Development Immersion

OVERVIEW

This is the recorded Drug Development Immersion course with the same content, interactive exercises, and course materials that are given in the live version. You have 3 months to view this course.

Drug Development Immersion is a nine-hour, interactive course that explores the regulatory, commercial, and scientific factors that enable a drug to be successfully brought to market. Discussion features both small molecule drugs and biologics. Our instructors illustrate the corporate decision-making process with personal accounts, giving participants unique insight into strategic development. Learn from an industry expert what it takes to get a molecule from the bench into the marketplace.

Drug Development Immersion was developed for the non-science professional who works within or services the biopharma industry and needs to become familiar with the preclinical and clinical development process.

Five takeaways

1. Fluency in essential terminology and acronyms used in clinical development.
2. In-depth look at the FDA and EMA regulatory process and sponsor interactions.
3. Criteria for preclinical studies to support first in human clinical trials.
4. Rationale, special considerations, and study design for both traditional and non-traditional clinical trial phases.
5. Understanding of the launch process, lifecycle management, and post-approval drug safety monitoring.

AGENDA

WEEK ONE

Setting the Stage 95 minutes

Small and large molecule drug characteristics
Desirable drug characteristics
Agonist and antagonist drugs
Route of administration based on drug type
Traditional drug development pathway
Gene and cell therapy development pathway
Drug development metrics
  Chances of success, timelines, and costs

WEEK TWO

The Business of Drug Development
  25 minutes

Integrated drug development process
Stage gates: go/no go decisions
Target product profile
Draft label
Activity: Draft Label
US patents and market exclusivity

WEEK THREE

The Players: Who is involved 40 minutes

Subjects, sponsors, investigators
Ethics committees/investigational review board
Informed consent
Contract research organizations
Patient advocacy groups
Data monitoring committees (DMC)
How DMC impacts clinical trials

WEEK FOUR

General Principles: Ethics and Risk
  25 minutes

Risk assessment and management
Bias and data integrity
Controlling bias: blinding and randomization

WEEK FIVE

The Regulatory Process 80 minutes

Regulatory agencies and compliance worldwide
PDUFA, GDUFA, BsUFA
Generics and biosimilars approval pathways
FDA/sponsor meeting timeline
FDA expedited programs
Voucher system
FDA and EMA orphan drug designation
EMA user fees and review times
EMA expedited reviews and designations
FDA and EMA approval process
Regulatory Compliance

WEEK SIX

Preclinical Development 80 minutes

Preclinical development pre-IND/CTA
Preclinical data objectives
Safety testing terms
Nonclinical studies
  Toxicology, pharmacology, pharmacokinetics
IND/CTA filings
Authorization to proceed to clinical trials

WEEK SEVEN

Conduct of Clinical Trials 75 minutes

Clinical research purpose
Introduction to study design elements
Endpoints
Inclusion/exclusion criteria
Placebos and control groups
Adverse events and safety reports
Clinical trial documentation
Data management and trial master files

WEEK EIGHT

Clinical Development Phase I

30 minutes

Purpose of Phase I
Design and conduct of Phase I
Selection of dose: MAD and SAD
Phase IA and IB
Bioequivalence trials
Combined Phase I/II studies
Combining Phase I/II trials

WEEK NINE

Clinical Development Phase II

25 minutes

Purpose of Phase II
Phase IIA and IIB
Randomized control trials
Statistical considerations
Null hypothesis, P value, type 1 and 2 errors
Activity: Introduction to Clinical Statistics

WEEK TEN

Clinical Development Phase III

50 minutes

Purpose of Phase III
Phase IIIB
Trial designs: parallel, crossover, adaptive
Database cleaning, lock, and unblinding
Regulatory application submittal

WEEK ELEVEN

Clinical Development Phase IV

15 minutes

Real-world evidence initiatives
Launch and lifecycle management
Drug safety and pharmacovigilance

Course Evaluation 20 minutes

The Regulatory Process for Drug Approval

OVERVIEW

The Regulatory Process for Drug Approval summarizes the regulatory bodies involved in approving both small molecule drugs and biologics. Understand the application process involved in moving a drug candidate from preclinical studies in animals to human clinical studies, to final approval allowing for the marketing and sale of a drug product. Learn how a drug candidate approval can be expedited so life-saving medicines can get to patients faster. Familiarize yourself with the US Prescription Drug User Fee Act (PDUFA) which governs it all. The Regulatory Process for Drug Approval gives an overview of all regulatory considerations a drug manufacturer should consider as it seeks to prove its drug is safe and effective.

Five Takeaways:

1. Discuss the components and timing of an Investigational New Drug (IND) application for the United States and a Clinical Trials Application (CTA) for the European Union.
2. Explain the purpose of the Prescription Drug User Fee Act of 1992 (PDUFA) and how it is a win/win for drug manufacturers, the FDA, and patients.
3. Understand the criteria of various expedited drug approval pathways and discuss how these designations effect the timing of the regulatory process.
4. Describe the process of filing for a New Drug Application (NDA) or Biologics License Application (BLA) in the United States and the Marketing Authorisation Application (MAA) in the European Union.
5. Compare and contrast generics and biosimilar approval pathways.

AGENDA

Introduction to the Regulatory Process

- Organization and mission of the FDA and EMA
- Global harmonization drug testing requirements

IND/CTA Filing

- IND applications and CTA
- Types and timing of IND filing

User Fee Programs

- PDUFA in conjunction with drug manufacturers, the FDA, and patients
- FDA and EMA interactions with industry

Orphan Drugs and Expedited Pathways

- Criteria for speeding up drug reviews
- Pathways employed by the EMA

Market Approval

- NDA and BLA in the US and lists the different
- Paths to approval in the EU
- Generics and biosimilars approval pathways

Drug Discovery of Small Molecule Drugs

OVERVIEW

Drug Discovery of Small Molecule Drugs explains the steps involved in discovering new therapeutics. This process includes early screening for targets, target validation, lead optimization, and determining when a target should be transitioned from discovery to development. Learn how new small molecule drugs are discovered and optimized before being tested in preclinical and clinical trials.

Five Takeaways:

1. In-depth knowledge of the drug discovery process.
2. Survey of typical discovery platforms.
3. Understanding of how to identify and validate a drug target.
4. Performance of lead optimization activities.
5. Criteria for the advancement of development candidates.

AGENDA

Overview

- Steps in drug discovery
- Screening considerations
- Bringing a new therapeutic to market

Early Screening

- Drug discovery platforms
- Target identification processes
- High throughput screening

Target Validation

- Target validation processes
- Target selection

Lead Optimization Criteria

- Screening strategies and pathways
- Lead optimization
- Drug design methods and approaches

Discovery to Development Transition Criteria

- In silico, in vitro, and in vivo data criteria
- Safety and efficacy

Drug Discovery of Biologics

OVERVIEW

Drug Discovery of Biologics explains the steps involved in discovering new biologics, with a special focus on therapeutic antibodies. Learn the criteria researchers use for early target screening, selection, validation, optimization, and determining when a drug target should be transitioned from discovery to development. Bonus content includes information on antibody diversity, affinity maturation, and key CMC challenges and how to overcome them.

Five Takeaways:

1. List the steps of discovery and lead optimization for biologics.
2. Explain target ID and selection considerations.
3. Explain how affinity maturation is tied to pharmaceutical liabilities.
4. Describe antibody diversity, IgG subtypes, and alternative formats.
5. Discuss the typical criteria for the advancement of development candidates.

AGENDA

Overview

- Drug discovery, development, and commercialization process
- Activities, costs and timing

Early Selection, Target ID, and IgG Subtypes

- Small and large molecule drug comparison
- Antibody production, selection, and humanization
- Target identification processes
- Antibody screening considerations

Affinity Maturation and Pharmaceutical Liabilities

- Structure based affinity maturation
- CMC liabilities in antibodies

Antibody Diversity and Alternative Formats

- Antibody therapeutic classes
- Immune checkpoint inhibitors
- Antibody classes in R&D

Discovery to Development and Transition Criteria

- Targeted decision making data analysis
- Transition criteria for biologic development candidates

Preclinical Development For Non-Scientists

OVERVIEW

Preclinical Development focuses on both small and large molecule drug safety assessments and regulatory requirements. This course also explains how clinical starting dose levels are estimated. Learn what preclinical criteria are needed to support first-in-human clinical trials.

Five Takeaways:

1. In-depth knowledge of the preclinical development process.
2. State the key data generated during pharmacology studies and why those data are collected.
3. Ability to estimate clinical starting dose levels by interpreting preclinical pharmacology and toxicology results.
4. Integration of preclinical data into the Common Technical Document.
5. Fluency of criteria necessary to support first-in-human clinical trials.

AGENDA

The Big Picture

- Development timing and costs
- In silico, In vitro, in vivo studies
- Safety and efficacy endpoints
- Preclinical short term studies
- Animal models

Pharmacology

- Pharmacology defined
- Pharmacology: antagonists and agonist drugs
- Pharmacology measurements
- Binding assay
- Potency assay
- Dose-response curves
- Receptor occupancy assay
- Efficacy assay

Pharmacokinetics and Pharmacodynamics

- Pharmacokinetics explained
- Measuring pharmacokinetics
- Pharmacokinetics: absorption
- Pharmacokinetics: metabolism
- Measuring pharmacodynamics
- Regulatory requirements
- GXP compliance
- Bioanalytical assay: small/large molecule drugs
- Validation timeline

Toxicology

- Preclinical design schedule
- Dose level selection
- Routes of exposure and formulation
- Species selection and three R's
- Concordance of animal and human toxicities
- Survey of main toxicology studies
- Therapeutic margin and adverse events
- Toxicology study data and its interpretation

Preclinical IND/CTA

- Common technical document
- IND and CTA filings
- Types of INDs
- FDA animal rule
- Interpretation

ON-DEMAND, SHORT CLASS | LEVEL ONE | 55-MINUTES

SUGGESTED PREREQUISITE: STRONG MOLECULAR BIOLOGY UNDERSTANDING RECOMMENDED

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Preclinical Development For Scientists

OVERVIEW

Preclinical Development for Scientists focuses on the FDA-required testing and safety assessments to gain IND approval. Real-world examples are used to explain how clinical starting dose levels are estimated based on dose-response curve results from pharmacology and toxicology studies. Real-world examples are used to explain how, based on animal studies, clinical starting dose levels are estimated. This course is for scientists with a strong molecular biology background.

Five Takeaways:

1. In-depth knowledge of the preclinical development process.
2. Fluency of FDA criteria necessary to support first-in-human clinical trials.
3. Ability to estimate clinical starting dose levels by interpreting preclinical dose-response curve results.
4. List of required assays, their purposes, and their criteria to move the candidate drug onto clinical trials.
5. Knowledge to integrate preclinical data into the Common Technical Document for an IND submittal.

AGENDA

Overview

- Drug development metrics: chances of success, timelines, and costs
- Preclinical development overview
- Investigational new drug (IND)
- Clinical trial application (CTA)
- Pre-IND: pharmacology, pharmacokinetics, toxicology

Pharmacology

- Pharmacology defined
- Binding assay: Law of Mass Action
  - Equilibrium dissociation (Kd)
  - Association /affinity (Ka) constants
- Potency assay: dose-response curves
- Efficacy assay: full, partial, inverse agonist and antagonist drugs
- Antagonist drug mechanism of action
- Agonist drug mechanism of action
- Product design: ROA, acute vs chronic, dose, container
- Animal model challenges

Pharmacokinetics

- Pharmacokinetics defined
- ADME
- Regulatory agencies
- GXP compliance: GLP, GMP, GCP
- Bioanalytical assay: API concentration
- Bioanalytical assay workflow and timeline
- Validation and qualification criteria
- Pharmacokinetics and pharmacodynamics measurements

Toxicology

- Animal model/species selection
- Animal research: replace, reduce, refine
- Concordance of animal and human toxicities
- Differences between animals and humans: subjects, doses, diagnostic procedures
- Preclinical testing: mutagenicity, hERG, acute/chronic toxcity, safety pharmacology, PK, PD, ADME, DART, carcinogenicity testing
- Therapeutic margin
- Adverse effects
- Example: 1 month rat study analysis

Nonclinical IND/CTA

- Filing IND/CTA
- Common technical document
- Module 2 summaries
- Module 4 reports
- FDA's animal rule

Clinical Development 101: General Principles

OVERVIEW

Clinical Development 101: General Principles sets the stage for the entire clinical development process. The general principles discussed include who conducts trials and their roles; how clinical trials are designed to reduce bias; why Good Clinical Practices help manage risk. Clinical Development 101 provides the foundational knowledge needed to understand the Clinical Trial Phases I-IV.

Five Takeaways:

1. Ability to explain the purpose of each clinical trial phase and list its milestone.
2. Familiarity with individuals and groups conducting a clinical trial and their roles.
3. Fluency in drug development concepts such as control groups, bias, blinding, randomization, and endpoints.
4. Knowledge of the study design criteria to reduce bias and keep patients safe.
5. Required regulatory studies and the data collected to enable follow-on studies.

AGENDA

Clinical Development Introduction

- Drug development milestones
- Clinical research and clinical studies
- Streamlining development in evidence-based medicine, translational medicine, and patient centric trials

Clinical Trials: Basic Principles

- Core principals of good clinical practices (GCP)
- Risk management in clinical trials
- Clinical trial designs

Conducting Clinical Trials

- Inclusion and exclusion criteria
- Ethics committees and institutional review boards
- Clinical trial data management and reporting

Clinical Development 201: Phase I

OVERVIEW

Clinical Development 201: Phase I explains the purpose of Phase I and the regulatory roles of the FDA, IRB, and EMA. Class highlights include how data from bioequivalence, pharmacokinetics, and pharmacodynamics studies are used to assess the success of Phase I endpoints. The last section explores how the single ascending dose and multiple ascending dose protocols help refine patient dosage schedules.

Five Takeaways:

1. Requirements for and maintenance of an Investigational New Drug (IND) application and a Clinical Trials Application (CTA).
2. Purpose of Phase 0 and Phase I clinical trials and the regulatory agencies that oversee the trials.
3. Expectations related to clinical benefit in early clinical trials for standard development programs, and development of treatments for conditions associated with serious unmet medical needs.
4. Typical endpoints assessed in Phase I clinical trials.
5. Steps sponsors take after Phase I is completed.

AGENDA

Clinical Trial Prerequisites

- Preclinical to Phase I clinical trials
- Clinical trial sequencing
- IRB, IB, IND, and CTA requirements

Phase 0/I Study Designs and Objectives

- Phase 0 and Phase I clinical trials
- Bioequivalence studies

Phase I Conducting the Clinical Study

- Maximum tolerated dose (MTD), single ascending dose (SAD), multiple ascending dose (MAD), pharmacokinetics, and pharmacodynamics data
- Endpoints assessed in Phase I clinical trials
- Clinical trial safety reports

Clinical Development 301: Phase II/III

OVERVIEW

Clinical Development 301: Phase II/III considers the purpose, design, and conduct of Phase II and III clinical trials. Learn the various trial design approaches, endpoint choices, statistical considerations, and special regulatory designations.

Five Takeaways:

1. Key differences between early stage (Phase I) and late-stage (Phase II/III) clinical trials.
2. Regulatory significance of clinical endpoint, primary endpoint, secondary endpoint, and surrogate endpoint.
3. Fluency in Phase II and Phase III clinical trial nuances.
4. Basic statistical analysis completed in late-stage trials.
5. Description of specialized and expedited development cycles for rare disease, orphan drugs, and therapies for unmet medical needs.

AGENDA

Phase II/III Introduction

- Transition from Phase I to Phases II and III
- Elements of a well controlled clinical trial
- Primary, secondary, and surrogate endpoints

Phase II/III Objective and Design

- Phase II and Phase III clinical trial characteristics and endpoints
- Pivotal study, adaptive trial, basket trial, and umbrella trial
- Data safety monitoring board function

Phase II/III Special Designations

- FDA orphan drug designations, EMA orphan drug status, and EU prime designation
- Clinical development for rare disease therapy

ON-DEMAND, SHORT CLASS | LEVEL THREE | 50-MINUTES

SUGGESTED PREREQUISITE: CLINICAL DEVELOPMENT 101, CLINICAL DEVELOPMENT 201,CLINICAL DEVELOPMENT 301

Clinical Development 401: Phase IV

OVERVIEW

Clinical Development 401: Phase IV surveys the ongoing post-approval clinical assessments required by regulatory agencies. Learn how drug risk management is accomplished through detecting, assessing and reporting adverse effects using real-world data.

Five Takeaways:

1. Purpose of Phase IV studies.
2. Key limitations of premarket studies and why post-market studies are an important complement to Phase I-III studies.
3. Role of regulatory safety information reporting programs including MedWatch in US and EudraVigilance in Europe.
4. In-depth look at real-world data and real-world evidence and their impact on safety.
5. Identification of important real-world data sources.

AGENDA

Post Approval Clinical Trials

- Key clinical milestones in drug development
- Phase IV clinical trials
- Limitations of Phase I-III clinical trials

Pharmacovigilance and Post-Marketing Safety Follow-Up

- Drug safety: pharmacovigilance
- Signal detection and analysis
- Regulatory actions: post-approval risk mitigation

Real-World Evidence

- Real-world evidence initiatives
- Real-world data supporting regulatory decision making