Design of pre-clinical experiments

Course: VPOCL2 | Department: Pharmacology

Duration: 30 Hrs | Coordinator: Dr. Divakar S

Who can enrol: B.Pharm, and PG students

Need/Purpose: The design of preclinical studies serves a crucial purpose in the drug development process, aiming to ensure the safety, efficacy, and feasibility of potential therapeutic drugs before they advance to clinical trials. The primary need for designing preclinical studies lies in assessing the feasibility and safety of drug compounds, as well as the effective and potential toxicities. These studies also play a vital role in characterizing the pharmacological activity of a drug compound, understanding its mechanism of action, and evaluating its absorption, distribution, metabolism, and excretion within the body. Moreover, preclinical studies are crucial for differentiating between exploratory (hypothesis-generating) and confirmatory (hypothesis-testing) research.

Learning objectives of the course:

- Understanding the fundamental principles of experimental design, including hypothesis formulation, variable selection, control group design, and statistical analysis.
- Learn to articulate research hypotheses and identify relevant outcome measures and endpoints.
- Learn how to select appropriate animal models or *in-vitro* systems that mimic the biological characteristics and disease pathophysiology relevant to the human condition.
- Learn how to conduct power analyses based on expected effect sizes, variability, and significance levels.
- Learn to develop standardized treatment protocols that specify drug doses, frequency, route of administration, and duration of treatment.
- Gain proficiency in selecting appropriate outcome measures to assess the safety and efficacy of the intervention.
- Gain proficiency in obtaining approval from institutional review boards or ethical review committees and adhering to principles of humane treatment and welfare.
- Learn to implement quality control measures to monitor and validate experimental procedures, equipment calibration, and data integrity throughout the study.
- Gain proficiency in maintaining comprehensive documentation of experimental procedures, data, and results in a detailed study protocol and laboratory notebook

Content/syllabus:

Module 1: Introduction to design of pre-clinical experiments and pre-clinical studies (Approx.10 hrs)

Setting clear research objectives and developing a testable hypothesis.

Sample size and grouping designing: Setting up sample size, designing control groups, randomization, and blinding strategies to minimize bias for statistical analysis.

Selection of appropriate outcome measures and data collection methods.

Definition and importance of pre-clinical studies in drug development.

Ethical considerations and regulatory frameworks governing pre-clinical research.

Introduction to scientific method and hypothesis-driven research.

The role of pre-clinical studies in safety assessment and efficacy evaluation.

Module 2: Strategy in selection of in-vitro and in-vivo models for hypothesis testing (Approx.10 hrs)

Differentiating exploratory (hypothesis generating) and confirmatory (hypothesis testing) research designs.

Strategies for target identification and validation in pre-clinical research.

Introduction to *in-vitro* and *in-vivo* models (cell lines, tissue cultures): Their applications, strengths, and limitations.

Route of administration and pharmacokinetics of drugs in animal models.

Principles of humane animal research (The 5Rs - Replacement, Reduction, Refinement, Reuse, and Rehabilitation) including regulatory requirements for animal research protocols.

Module 3: Statistical analysis of pre-clinical data for communication and reporting (Approx.10 hrs)

Statistical concepts relevant to pre-clinical research (descriptive statistics, hypothesis testing).

Choosing the appropriate statistical tests based on data type and study design.

Writing clear and concise scientific reports, interpreting statistical results and drawing conclusions from pre-clinical data.

Effective presentation of pre-clinical study design, methods, and results.

Addressing the ethical considerations and limitations of the study.

Teaching & Learning activities

Theory – 08

Demonstration and Hands-on Training – 16 hours

Assessment – 06 hours