PHARM.D • YEAR 5

Clinical Research

CHAPTER 2

Clinical Development of a Drug

PPT CHAPTER COVER



Clinical trials: definition and purposes

Clinical trials study interventions in human participants under a defined research plan.

They evaluate efficacy, safety and a suitable dose or regimen.

Comparisons may involve standard treatment or an ethically justified placebo.



Some trials address prevention, screening, treatment or supportive care and quality of life.

Additional studies examine long-term effects or use in specific populations.

Trial evidence supports clinical decisions and regulatory assessment.

Intervention trial



Participants are assigned an intervention and outcomes are measured and compared (e.g. standard treatment or an ethically justified placebo).

Observational study



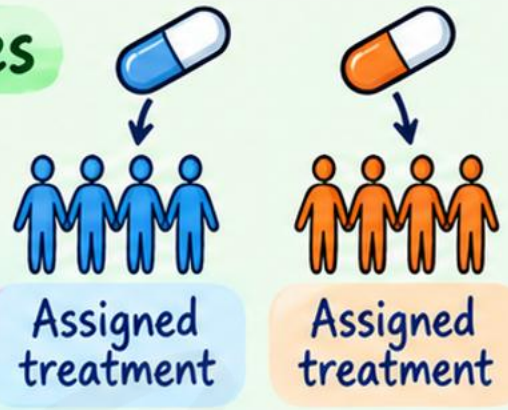
An observational study records routine exposure and outcomes without assigning the intervention.

Trial evidence supports clinical decisions and regulatory assessment.

Trial designs, ethics and participants

Interventional studies

assign a treatment or other intervention.



Observational studies

follow exposure and outcomes without investigator assignment.



Observed routine care

Informed consent

supports a voluntary, informed choice.



Independent IRB/IEC

reviews the ethical acceptability of the research.



Participants



Participants enter according to predefined inclusion and exclusion criteria.

The sponsor

initiates and manages the trial. Investigators conduct it at sites.



CROs

provide contracted services.



Regulators

oversee applicable trial and product requirements.



GCP

supports participant protection and reliable results.



Exploratory trials and Phase 0

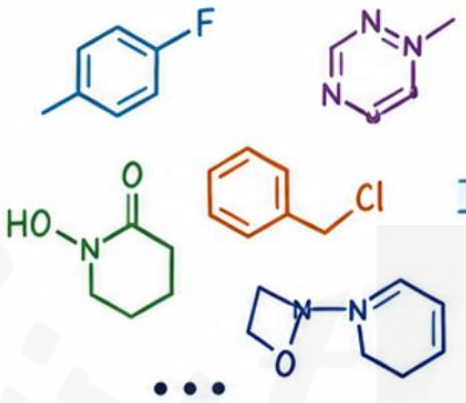
Exploratory trials

Exploratory studies obtain limited early human information before conventional therapeutic development.

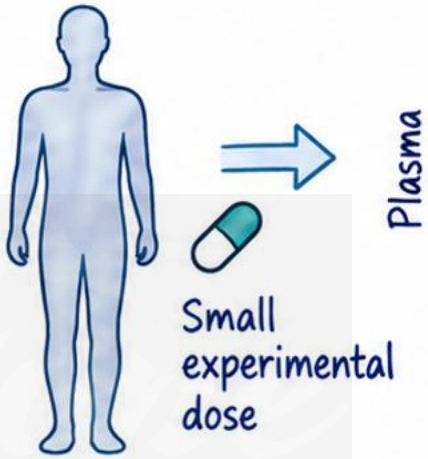
They can examine PK, target engagement or preliminary PD and help select a candidate.

Exposure and duration are limited, with no therapeutic or diagnostic intent.

From many molecules ...

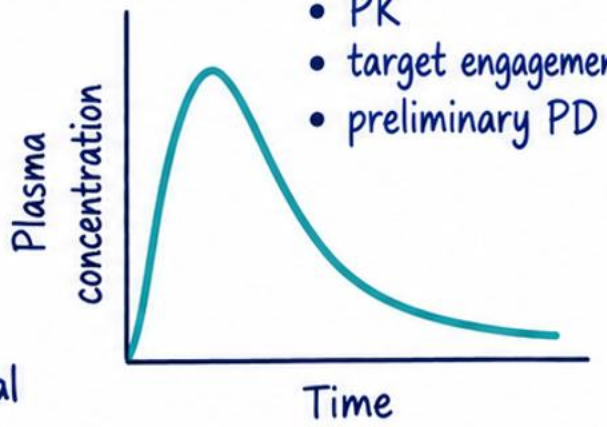


Molecule screening to select a candidate



Small experimental dose

Early human information



Microdosing



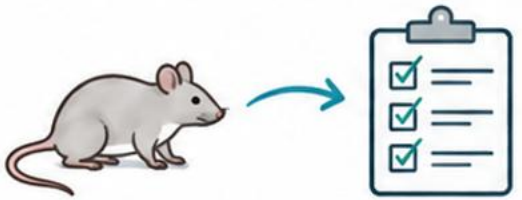
Microdosing is one exploratory approach using very small doses.

Example (not a universal rule)



Some Phase 0 studies involve fewer than 15 participants, but this is an example rather than a universal rule.

Nonclinical support



Nonclinical support must match the proposed exposure.

Phase 0 is optional

Phase 0 is optional and does not replace conventional safety evaluation.

Phase I: initial clinical evaluation

Main aims: initial safety, tolerability, PK and PD.

Common scale: about 20–100 participants, often over several months.

Healthy volunteers are common, but patients may participate when safety or the indication requires it.

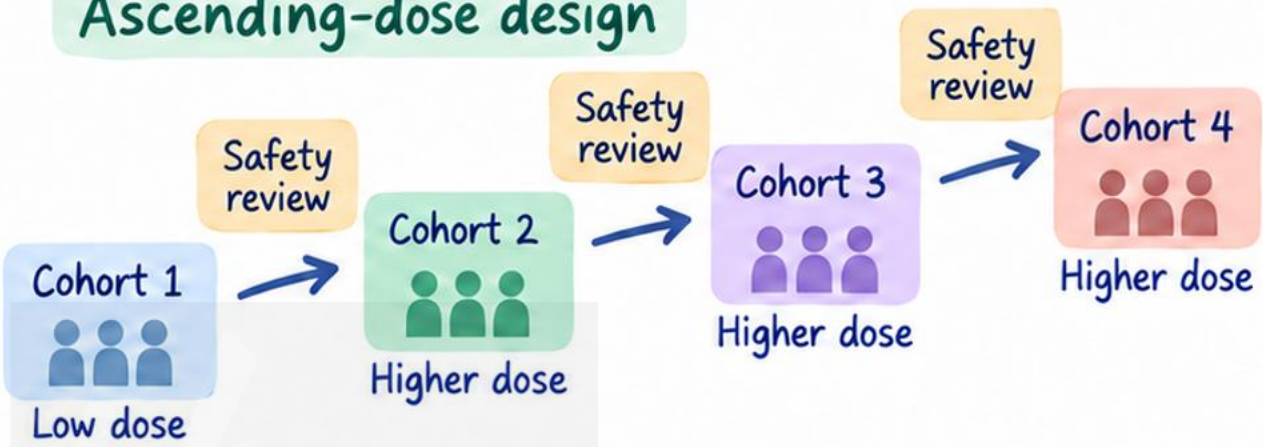
Studies assess ADME, half-life, adverse effects and dose-exposure relationships.

Single or multiple ascending-dose designs may guide a suitable dose range.

MTD is relevant to some programs, particularly oncology, rather than every drug.

Designs may include randomization and blinding. Numbers and duration vary.

Ascending-dose design



Healthy volunteers

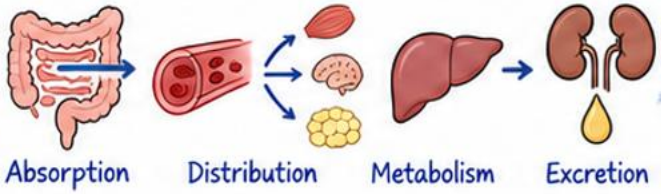


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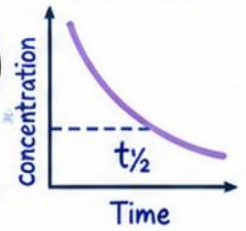
Patients



ADME



Half-life



Adverse effects



Dose-exposure relationships



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