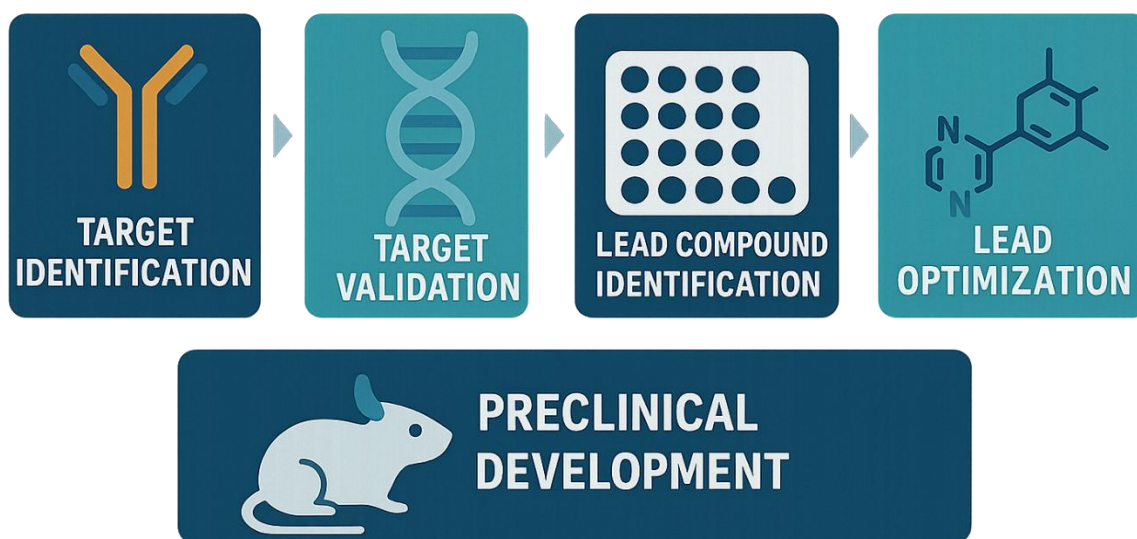


New Drug Discovery and development

Stages of Drug Discovery

- Drug discovery is the scientific process of identifying new candidate medications.
- It represents the earliest phase in the lifecycle of a drug and focuses on finding molecules that could potentially be developed into therapeutic products.
- This phase can span several years and involves intensive research and validation.

STAGES OF DRUG DISCOVERY



1. Target Identification

- Researchers identify biological targets involved in a specific disease.
- Targets are typically proteins such as enzymes, receptors, or ion channels.
- The goal is to find a target whose modulation (activation or inhibition) can provide a therapeutic benefit.

Key Methods:

- Genomic and proteomic analysis
- Literature reviews and bioinformatics databases
- Disease pathway mapping

2. Target Validation

- Confirms that the chosen target is causally linked to the disease process.
- Demonstrates that modulation of the target produces a therapeutic effect.

Approaches Include:

- Genetic manipulation (e.g., gene knockouts or knockdowns)
- Use of monoclonal antibodies or RNA interference (RNAi)

- Functional studies in animal models

3. Lead Compound Identification (Hit Identification)

- Researchers seek compounds that interact effectively with the validated target.
- This step identifies "hit" compounds using various screening techniques.

Methods Include:

- High-throughput screening of chemical libraries
- Virtual screening using computational models
- Natural product screening

Outcome:

- A list of "hit" compounds that show activity against the target in in vitro assays.

4. Lead Optimization

- Refinement of hit compounds to improve their drug-like properties.
- Focus is on improving:
 - Potency
 - Selectivity
 - Pharmacokinetics (PK)
 - Safety profile

Techniques Used:

- Structure-Activity Relationship (SAR) studies
- Quantitative SAR (QSAR) analyses

5. Preclinical Testing

- Promising lead compounds are tested for safety and biological activity before human trials.

Objectives:

- Assess safety and biological activity
- Determine pharmacokinetics (PK) and pharmacodynamics (PD)
- Evaluate toxicology and safety pharmacology

Includes:

- In vitro (lab-based) testing
- In vivo (animal-based) testing
- Toxicity studies (acute, sub-chronic, chronic)
- Dose-ranging and mechanism-of-action studies

Drug Development Process

Once a compound shows promise in pre-clinical studies, it enters the drug development phase, which includes clinical trials and regulatory approval.

1. Investigational New Drug (IND) Application

- Submitted to regulatory authorities (e.g., FDA in the U.S.) before clinical trials begin.
- Contains preclinical data, proposed clinical study protocols, manufacturing info, and investigator info.

2. Clinical Trials

- Clinical trials are conducted in **three main phases** to test safety and efficacy in humans:

Phase I

- **Participants:** 20–100 healthy volunteers or patients.
- **Purpose:** Assess safety, dosage range, and pharmacokinetics.
- **Duration:** Several months.

Phase II

- **Participants:** 100–300 patients with the disease.
- **Purpose:** Evaluate efficacy and side effects.
- **Design:** Randomized controlled trials.
- **Duration:** Months to 2 years.

Phase III

- **Participants:** 1,000–3,000 patients.
- **Purpose:** Confirm effectiveness, monitor side effects, and compare to standard treatments.
- **Design:** Double-blind, randomized.
- **Duration:** 1–4 years.

3. New Drug Application (NDA) / Marketing Authorization Application (MAA)

- Submitted after successful clinical trials.
- Reviewed by regulatory authorities for approval.
- Includes all data from preclinical and clinical studies, manufacturing info, and proposed labeling.

4. Regulatory Review and Approval

- Regulatory agencies review the NDA/MAA.
- If approved, the drug is allowed for marketing.



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