

Drug discovery is the process of identifying and developing new pharmaceutical compounds to treat diseases. It involves multiple stages, from target identification to clinical trials and regulatory approval. Below is an overview of the key steps in drug discovery:

1. Target Identification & Validation - Identify a biological molecule (e.g., protein, enzyme, receptor) that plays a crucial role in a disease. - Validate its role in disease progression through experimental and computational methods.

2. Hit Discovery & Lead Identification - High-Throughput Screening (HTS): Test thousands to millions of compounds to identify "hits" that interact with the target. - **Structure-Based Drug Design (SBDD):** Use computational modeling (e.g., molecular docking) to design molecules that bind effectively. - **Fragment-Based Drug Discovery (FBDD):** Identify small molecular fragments that bind to the target and optimize them into larger drug candidates.

3. Lead Optimization - Modify and refine lead compounds to improve potency, selectivity, and pharmacokinetic properties (absorption, distribution, metabolism, excretion). - Reduce toxicity and off-target effects.

4. Preclinical Testing - In Vitro Testing: Assess biological activity in cell-based assays. - **In Vivo Testing:** Conduct animal studies to evaluate safety, efficacy, and metabolism.

5. Clinical Trials - Phase 1 (Safety & Dosage): Small group of healthy volunteers to determine safety and pharmacokinetics. - **Phase 2 (Efficacy & Side Effects):** Larger group of patients to evaluate therapeutic effects and side effects. - **Phase 3 (Large-Scale Trials):** Thousands of patients in multi-center trials to confirm effectiveness and monitor adverse reactions.

6. Regulatory Approval - Submit a New Drug Application (NDA) or Biologics License Application (BLA) to regulatory agencies (e.g., FDA, EMA). - Review process assesses the drug's safety, efficacy, and manufacturing quality.

7. Post-Market Surveillance - Monitor long-term safety and effectiveness in the general population. - Report adverse effects and update treatment guidelines if necessary.

Modern Approaches in Drug Discovery - Artificial Intelligence (AI) & Machine Learning (ML): Predict drug-target interactions, optimize compounds, and analyze clinical data. - **CRISPR & Gene Editing:** Target genetic factors contributing to diseases. - **mRNA-based Therapeutics:** Inspired by COVID-19 vaccines, mRNA drugs are being developed for various diseases. - **Proteolysis-Targeting Chimeras (PROTACs):** Degrade disease-causing proteins rather than just inhibiting them.

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