

Overview of Modern Drug Discovery Process

Target Identification

- Target identification is the first step in modern drug discovery.
- It involves finding a biological molecule in the body that plays a key role in a disease and can be affected by a drug.

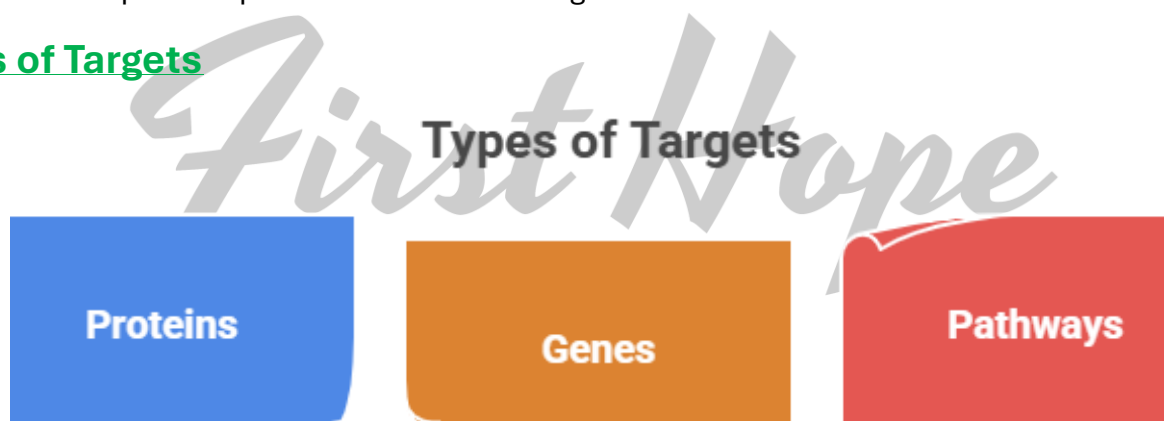
What is a Target

- A target is usually a protein such as an enzyme, receptor, or ion channel.
- It can also be DNA or RNA in some cases.
- The target is linked to the cause or progression of a disease.

Why Target Identification is Needed

- To understand the cause of the disease
- To find a point where a drug can act
- To develop more specific and effective drugs

Types of Targets



1. Proteins

- Enzymes that control chemical reactions
- Receptors that receive signals in the body

2. Genes

- Mutated genes that cause diseases
- Genes that control protein production

3. Pathways

- Biological pathways involved in disease processes

Methods Used for Target Identification

1. Genomic Studies

- Study of genes to find disease-related changes

2. Proteomics

- Study of proteins and their functions

3. Bioinformatics

- Use of computer tools to analyze biological data

Example

- In cancer, abnormal proteins that control cell growth can be targets

Importance

- It gives direction to the entire drug discovery process.
- A wrong target can lead to failure of the drug.

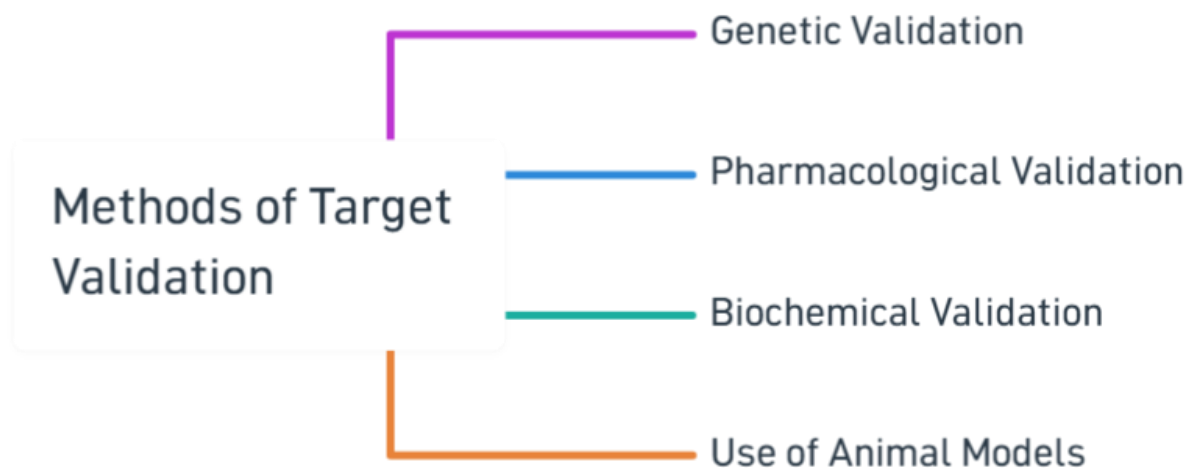
Target Validation

- Target validation is the process of confirming that the identified target is truly involved in the disease and can be safely used for drug development.

Why Target Validation is Needed

- To ensure the target is relevant to the disease
- To confirm that acting on the target will give a therapeutic effect
- To reduce failure in later stages

Methods of Target Validation



1. Genetic Validation

- Scientists modify or remove the gene related to the target.
- They observe whether the disease condition changes.

2. Pharmacological Validation

- Known drugs or molecules are used to affect the target.

- The effect on disease symptoms is studied.

3. Biochemical Validation

- The activity of the target is measured in laboratory experiments.
- Researchers check how it behaves under different conditions.

4. Use of Animal Models

- The target is tested in animals to see real biological effects.
- This helps in understanding safety and effectiveness.

Types of Validation

1. In vitro Validation

- Experiments done in cells or tissues in the laboratory

2. In vivo Validation

- Experiments done in living organisms like animals

Key Points

- A validated target increases the chance of successful drug development
- Poor validation can lead to failure in clinical trials

Importance

- It confirms that working on this target will help treat the disease.
- It saves time and cost by avoiding unsuitable targets.

Lead Identification

- Lead identification is the process of finding chemical compounds that can interact with the validated target and show some desired biological effect.
- These compounds are called "lead compounds."

Why Lead Identification is Needed

- To find starting molecules for drug development
- To discover compounds that can affect the target
- To select promising candidates for further improvement

Sources of Lead Compounds

1. Natural Sources

- Plants, microorganisms, and marine organisms
- Many drugs are originally derived from natural products

2. Synthetic Compounds

- Chemicals made in laboratories
- Designed to interact with specific targets

3. Existing Drugs

- Drugs already in use for other diseases
- Can be modified or repurposed

Methods of Lead Identification

1. High-Throughput Screening (HTS)

- Thousands of compounds are tested quickly.
- Machines help in identifying active compounds.

2. Virtual Screening

- Computer models are used to predict which compounds may bind to the target.
- This saves time and resources.

3. Natural Sources

- Compounds are obtained from plants, microbes, or marine sources.
- Many drugs originate from natural products.

4. Fragment-Based Screening

- Small chemical fragments are tested.
- These fragments are later combined to form a strong lead compound.

Characteristics of a Good Lead Compound

- Shows activity against the target
- Has acceptable safety in early tests
- Has a chemical structure that can be modified

Importance

- It provides the starting point for drug development.
- Without a good lead, further steps cannot proceed effectively.

Lead Optimization

- Lead optimization is the process of improving the lead compound to make it more effective, safe, and suitable as a drug.

Why Lead Optimization is Needed

- To increase drug potency (strength of effect)
- To reduce side effects and toxicity
- To improve how the drug behaves in the body

Parameters Improved During Optimization

1. Potency

- The strength of the compound to act on the target is increased.
- Small chemical changes are made to improve binding.

2. Selectivity

- The compound is modified to act only on the desired target.
- This reduces unwanted side effects.

3. Pharmacokinetics (ADME)

- Absorption, distribution, metabolism, and excretion are improved.
- The drug should stay in the body for an appropriate time.

4. Toxicity Reduction

- Harmful effects of the compound are minimized.
- Safer versions of the molecule are developed.

Methods Used in Lead Optimization

1. Structure-Activity Relationship (SAR)

- Scientists study how changes in structure affect activity.
- This helps in designing better compounds.

2. Medicinal Chemistry

- Chemical modifications are made in the laboratory.
- New versions of the lead are synthesized and tested.

3. Computer-Aided Drug Design (CADD)

- Computer models help in predicting better structures.
- This speeds up the optimization process.

Outcome of Lead Optimization

- A refined compound with better efficacy and safety
- Ready for preclinical testing

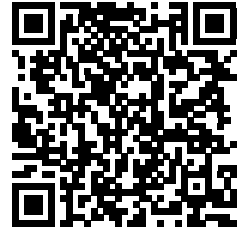


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