DRUG DISCOVERY & DEVELOPMENT: DISCOVERY, DEVELOPMENT & REGULATORY

On average, out of **10,000 COMPOUNDS** tested, **250** will move to pre-clinical stage; **5** of these compounds will move to clinical trials, and ultimately **1** will receive FDA approval. The full research, development and approval process can take **12-15 YEARS** or longer.

Developing a new prescription medicine that gains marketing approval is estimated to cost drugmakers **$2.6 BILLION** according to a recent study by Tufts Center for the Study of Drug Development.

Discovery

The drug development process begins with discovery, the preclinical research testing of molecular compounds.

This first stage includes:

- Identifying a target and screen a library of compounds using robotics
- Conduct experiments with the best compounds identified to select those that that exhibit the desired outcome in cells (in vitro)
- Conduct experiments using the selected compounds in living organisms to assess if they improve the symptoms (in vivo)

When a promising compound is identified, researchers conduct experiments to gather information and design clinical trial protocols.

Development & Regulatory

An Investigational New Drug (IND) application is submitted to the FDA.

An IND includes:

- Safety and efficacy data from research studies using the compound in cells and animals
- The compound composition and manufacturing
- A proposed plan for testing the compound in clinical trials in people

When permission is given by the FDA to begin clinical trials:

Phase 1
Study in healthy volunteers closely monitoring and gathering information about how a drug interacts with the human body, its safety and way the body breaks down the drug.
Approximately 70% of drugs move to Phase 2

Phase 2
Study with patients with disease/condition to provide additional safety data.
Approximately 33% of drugs move the Phase 3

Phase 3
Study with expanded patient population monitoring long term safety and efficacy.
Approximately 25-30% of drugs move forward.

After a drug receives FDA approval a **Phase 4** study is conducted for post-market safety monitoring.

Regulatory

New Drug Application (NDA) is the formal step asking the FDA to consider a drug for marketing. It tells the full story of a drug and its purpose is to demonstrate that a drug is safe and effective.

Advisory Committee is a body of experts, including patients, that the FDA enlists for consultation when they deem it necessary.

The FDA reviews study data and decides whether to grant approval or not. Additional research or an expert advisory panel may be required before a final decision is made by the FDA.

What Does FDA Approval Mean?

The FDA's drug review process aims to ensure drugs are safe, effective, and manufactured properly.

FDA approval means that the data on the drug's effect have been determined to provide benefits that outweigh its known and potential risks for the intended population.

Connect with resources to help guide you through this journey

1-833-PTC-HOPE (1-833-782-4673), or in Spanish 1-833-PTC-VIDA (1-833-782-8432)

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