



The Drug Development Cycle ~ An Overview

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For venture capital firms, the potential rewards of investing in life science companies can be enormous. However, reward often comes with a price: risk, and emerging biotech and drug companies are no exception to the rule. The process of developing a new drug can take between 10 and 15 years with an estimated cost of \$800 million¹. This developmental process ensures that drugs brought to the market are safe when used appropriately, and effective when used against the targeted disease or medical disorder. Satisfying these two objectives involves a series of developmental stages, from the laboratory bench to the marketplace.

Discovery/Pre-Clinical Testing

Time: 6.5 Years

Discovery

The process of drug development begins with the discovery of potential drug candidates. After screening through thousands of compounds that may have an effect on a location in the body (target), a few are selected and optimized to enhance the impact of the potential drug against the target. Promising drug candidates are then entered into the Pre-Clinical research phase.

Pre-Clinical

Before a drug can be tested in humans, it must be tested for effectiveness in laboratory and animal studies. Animal studies are conducted to determine if there are any harmful effects of the drug and to help understand how the drug works. If these studies show that the experimental drug may be effective in treating/preventing a specific disease or medical disorder, the drug company will file an Investigation New Drug Application (IND) with the FDA. The IND must also explain how the new drug works, why it may be useful in treating a disease, and what side effects the drug is known to cause.

Phase I

Time: 1.5 Years

The primary goal of a Phase I trial is to determine the safety of a drug. Phase I trials are typically conducted on relatively small groups of healthy volunteers that are closely monitored by a clinical team. The studies are designed to assess the drug's safety, its pharmacokinetics (what the body does to the drug), and its pharmacodynamics (what the drug does to the body).

Phase II: Safety and Efficacy

Time: 2 Years

Upon completion of the Phase I trials, a drug company will then proceed to the larger Phase II trials to assess whether the drug is effective in treating or preventing the targeted disease or medical disorder. In order to assess the drug's efficacy, patients at this stage have usually been diagnosed with the targeted medical condition. Other aims of Phase II trials include determining an effective dose that minimizes adverse side effects and identifying any potential interactions with other drugs.

Phase II trials are usually double-blinded placebo controlled studies, where neither the investigator nor the patient know if the patient is being administered the active drug or a placebo. If the Phase II trials have been well-designed, the company can then confidently make the critical decision of whether to proceed with the more expensive Phase III trials.

Phase III:

Time: 3.5 Years

Phase III is where a drug's safety and efficacy are verified in a large and varied clinical population. Anywhere from hundreds to thousands of patients participate in these studies that are conducted at multiple sites. These trials are randomized and usually placebo-controlled (unless using a placebo would be unethical).

The purpose of this larger pool is to enable investigators to identify adverse drug reactions and to determine the appropriate dosage for a more diverse general population. Fulfilling this objective however, is very costly due to the large number of patients that must be recruited, clinical monitoring costs, compensation for medical professionals, and any hospitalization fees that may be required.

Marketing Approval Process

Time: 1.5 Years

If the Phase III trials prove efficacy and safety, the company can then submit an application to the FDA to market the drug. This application must contain all the clinical data compiled during testing of the drug.

The FDA takes about 1 year to review a typical application of a "standard" product. For "priority" therapeutics that are targeted for life-threatening illnesses or an unmet medical need, the FDA has 6 months to review and respond to the drug application.

The FDA may approve the proposed labeling, approve modified labeling, send the sponsor back to conduct additional trials, or may refuse approval outright. Sometimes the FDA will give conditional approval but require additional post-marketing trials to answer specific additional efficacy or safety questions.