

## **BACKGROUNDER: How New Drugs Move through the Development and Approval Process**

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BOSTON – (November 2001) – Between the time research begins to develop a new prescription medicine until it receives approval from the Food and Drug Administration (FDA) to market the drug in the United States, a drug company typically spends \$802 million over the course of 10 to 15 years.

Here's why it costs so much and takes so long.

Of every 5,000 medicines tested, according to the Pharmaceutical Research and Manufacturers of America, only five on average are tested in clinical trials. Based on research by the Tufts Center for the Study of Drug Development, only one of these five is eventually approved for patient use,.

Drug development in the United States follows a number of steps carefully defined by the FDA. Here's a brief overview.

New drugs are developed by drug sponsors, which may be an individual, partnership, corporation, government agency, manufacturer, or scientific institution which will market the new drug. The sponsor first engages in a discovery process to identify molecules for further development.

Once identified, the sponsor notifies the FDA of its intent to conduct clinical studies on human subjects. This is called filing an initial new drug application, also referred to as an IND. The IND involves a detailed review process, which, if successful, paves the way to clinical studies. Clinical development typically involve three phases of study, as follows.

Phase I studies may be conducted in patients, but typically involve healthy volunteer subjects. These studies are designed to determine the pharmacokinetic (how the drug is absorbed, distributed, metabolized, and excreted by the body) and pharmacologic (the effect of the drug on the body) actions of the drug in humans, the side effects associated with increasing doses, and, if possible, early evidence on effectiveness.

Upon conclusion of Phase I studies, the company determines whether results are promising enough to pursue a Phase II study to help determine the scientific validity of the drug. If the answer is negative, research on the compound is terminated.

Phase II studies are designed to obtain data on the effectiveness of the drug for a particular indication or indications in patients with the disease or condition. They also help determine the common short-term side effects and risks associated with the drug. Phase II studies are closely monitored and conducted in a relatively small number of patients. At this point, the company again evaluates whether it should pursue further research on the molecule. A positive decision will lead to Phase III studies.

Phase III studies are expanded controlled and uncontrolled trials. They are done to gain additional data about effectiveness and safety needed to evaluate the benefits and risks of the drug. Results from Phase III studies also yield data that will provide the information that eventually will go on physician labeling.

The number of human subjects involved progressively increases from phase to phase, with Phase III studies typically including several hundred to several thousand people.

Overseeing the clinical studies is the FDA's Center for Drug Evaluation and Research, known as CDER, which can halt the studies if it deems them unsafe or if it believes their design will not meet the study's stated objectives. CDER engages outside expert advisors to help review study results.

While clinical studies often take many years to complete, it is possible to accelerate patient's access to the drug under FDA's expedited development and review programs. These programs are designed to speed the availability of those drugs that promise significant benefits over existing treatments for serious or life-threatening diseases for which no adequate therapies exist.

#### About the Tufts Center for the Study of Drug Development

Based in Boston, Mass. and affiliated with Tufts University, the Tufts Center for the Study of Drug Development (<http://csdd.tufts.edu>) provides strategic information to help drug developers, regulators, and policy makers improve the quality and efficiency of pharmaceutical development, review, and utilization. The Tufts Center conducts a wide range of in-depth analyses on pharmaceutical issues and hosts symposia, workshops, and public forums on related topics throughout the year.

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