Developing Medicines

It's sometimes hard to remember that just a generation ago, there were few effective treatments for cancer, AIDS, heart disease, stroke or many other serious medical conditions. Since 1990, 300 medicines have been approved in the U.S. alone, to treat more than 150 diseases. While we still have a long way to go to banish the most devastating diseases, there's been enormous progress.

Today, many patients can be treated with a medicine instead of a long and costly hospital stay. New medicines extend lives, improve the quality of life and delay nursing home care.

Creating new medicines is a challenging, costly, high-risk undertaking, and one that can take more than 15 years.

Potential new medicines pass through several crucial stages on their way from the research laboratory to the pharmacy shelf. Only a handful – just one in every 10,000 substances tested – will survive the rigorous process and be approved for patient use. Besides taking many years, it's been estimated to cost more than $800 million dollars in R&D for each new medicine approved.[1]

Discovery Research

The search begins with basic research in a laboratory. Scientists focus on specific diseases and the associated areas of the body where medications may ease or correct the problem. They review thousands of compounds to test for potential biological activity.

Producing a promising molecule is only the first step in a long process. With any new potential drug, researchers must figure out:

- How to deliver the new compound to the specific body target
- How natural barriers in the body might interact with the compound
- Whether the medicine is delivered orally or by injection
- What dose will prove effective
- What side effects are possible
- Whether the new compound can be reproduced successfully in a manufacturing setting for a reasonable cost.

1 Tufts Center for the Study of Drug Development, Tufts University.
Available at www.ncbi.nlm.nih.gov/sites/entrez?db=pubmed&uid=12606142&cmd=showdetailview&indexed=google

Answering these questions is just the beginning. Then potential new medicines are put through a vigorous set of tests.

The first test is the "pre-clinical trial." Tests are conducted in laboratory dishes ("in vitro research") or in animals to find out if the drug is active against the disease, how it works and whether and how it affects any other body systems. Once the pre-clinical studies are done, clinical trials begin in people. Clinical studies are one of the most important steps in the research process. If a medicine passes through the various steps or phases of clinical research and demonstrates both effectiveness and safety, it is submitted to the appropriate government agencies for review.

Clinical Studies

Human clinical studies follow in a series:

- Phase I clinical studies, with a small number of healthy volunteers, establish the compound's basic safety profile in people.
- Phase II clinical studies involve tests with small numbers of patients who have the disease being investigated to see how effective the drug is when given to people and to further understand safety.
- Phase III clinical studies involve tests with large numbers of patients who have the disease. These studies are large enough to confirm that a drug works or does not work and whether it has safety issues that are common. Phase III trials cannot typically find extremely rare side effects. These typically show up only after many thousands of patients have taken the medicine because they are so rare.
- Phase IV, or post-market, clinical studies provide more information about the safety and effectiveness of already-approved health care products. Legislation signed in 2007 further gives the U.S. Food and Drug Administration authority to require phase IV studies.