

The Research and Development Pipeline: A Primer

The Basics

What: The pharmaceutical research & development (R&D) pipeline is the process for identifying a potentially beneficial drug, proving that it is safe and effective, and making it available in a way that maximizes its benefit to as many patients as possible.

Who: The pharmaceutical industry is the largest funder of the pipeline, accounting for approximately 70% of U.S. investment in pharmaceutical R&D. The federal government, particularly the National Institutes of Health, is the second largest funder at about 20%. Research institutions, industry, government, and patients each play a role in executing the multiple phases of pharmaceutical R&D.

Why: The pharmaceutical R&D pipeline is a process of human effort and accomplishment that conveys profoundly important benefits to society across time and generations. Hundreds of millions of people have lived longer and healthier lives because of vaccines and other preventative measures, treatments and cures. And medical progress is not an exhaustible commodity; with every new discovery, the scope and societal value of medical progress grows.

That said: It is an imperfect pipeline – inefficiencies, public and private sector investment gaps, and other challenges exist. Further, medical progress itself leads to important public policy issues including health care affordability and universal access.

So: Policymakers have a responsibility to address issues related to pharmaceutical R&D and medical progress. To reach sound policy solutions, it is critical to fully value the role the pharmaceutical R&D pipeline and medical progress play in the longevity, health, and wellbeing of us all.

The Process

Step 1: Basic Research

Basic research seeks to shed light on fundamental questions about living organisms (the what, why, when, and how). The discovery of the DNA double helix is a prime example of basic research because scientists sought to reveal knowledge without focusing on a specific health need. Scientists set out to determine DNA's structure, and their success established the foundation for subsequent years of ground-breaking applied research across different diseases and disorders.

Step 2: Applied Research

Applied research begins after the identification of a potential path forward to address a health concern. For example, basic research on the structure and composition of DNA has set the stage for applied research to uncover mutations in DNA that could cause disease. With this information, scientists are conducting further applied research specific to these mutations to better understand and treat different diseases.

- **Preclinical Research:** Preclinical research often occurs in parallel with applied research. It includes targeted drug testing in animal models and is completed prior to clinical trials in humans.

Step 3: Clinical Research

Once applied research identifies potential medical advances, clinical research further refines and assesses these potential advances. Clinical research typically involves both patients and healthy volunteers and is completed in 4 (sometimes 3) phases:

- **Phase I** examines the safety of the product in a very small group of healthy volunteers. Approximately 70% of drugs make it past Phase I.
- **Phase II** assesses the efficacy and correct dosing in a larger group of patients. Approximately 33% of drugs make it past this stage.
- **Phase III** tests the product in a much larger, more diverse population to confirm efficacy, monitor potential side-effects, and develop usage guidelines. Up to 30% of drugs make it past this point.
- **Phase IV**, otherwise known as Post-Market Safety Monitoring, assesses continued safety in the first few months to years after a drug is in use by patients.

The Role of Food and Drug Administration Regulatory Review

The [Food and Drug Administration](#) (FDA) is responsible for reviewing new drugs to determine whether they can be marketed in the U.S. FDA reviews data on safety and efficacy and consults with outside experts to determine whether the benefits of the new drug outweigh any risks associated with its use.

Upon a drug's approval, the agency works with the applicant on drug labeling – the process of developing and refining prescription information – and companies begin the process of scaling up production.

Implementation in Health Care

As a new drug enters the market, practitioners must determine when and how to use it in real world healthcare settings. The [Agency for Healthcare Research and Quality](#) and the National Institutes of Health fund health services research and implementation research to promote best practices as new medical advances, including new medicines, are incorporated into the practice of medicine.

The Role of Technology Transfer

Technology transfer is the movement of intellectual property (IP) rights between individuals or organizations. The landmark, bipartisan Bayh-Dole Act, signed into law in 1980, [gives universities the rights to intellectual property \(IP\) generated from federal funding](#). By fostering academic-industry partnerships in the applied, pre-clinical, and clinical research phases of drug development, Bayh-Dole ushered in a new era of cross-sector collaboration and medical progress.

Additional Resources

Resources that provide a more in-depth look at the topics covered in this primer:

1. [Research and Development Pipeline Infographic](#)
2. [Milken Institute Medical Research and Development Primer](#)
3. [Congressional Budget Office \(CBO\) Research & Development in the Pharmaceutical Industry](#)
4. [U.S. Food and Drug Administration Drug Development & Approval Process](#)
5. [Additional Data on U.S. R&D Spending by Sector](#)
6. [Pharmaceutical R&D in the Global Context](#)
7. [Learn More About Technology Transfer](#)
8. [The Role of the Agency for Healthcare Research and Quality](#)