The Dynamic U.S. Research and Development Ecosystem

The rapid pace of scientific and technological advances and our growing understanding of the underlying mechanisms of disease are fueling the development of new treatments and cures for patients. In turn, scientific, technical and regulatory challenges related to the drug development enterprise create complexities as companies often focus their research and development (R&D) where the science is most complex and the failure risks are higher. The impressive progress in COVID-19 therapeutics and vaccines reflects the tireless efforts and commitment of America's research-based biopharmaceutical companies working with public partners and others to combat this global threat. The U.S. R&D ecosystem continues to leverage new scientific and technological advances to bring innovative medicines to patients. With more than 8,000 medicines in clinical development globally—of which 74% have the potential to be first-in-class treatments—the future has never been more promising.[1,2]

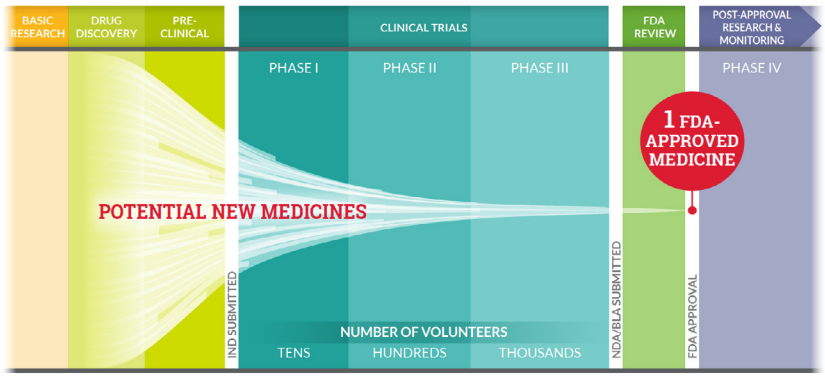
America's Biopharmaceutical Companies Play Central Role in R&D Ecosystem

America's biopharmaceutical companies are at the heart of a robust research and development (R&D) ecosystem that develops more innovative medicines than any other country in the world. In recent years, rapid advances in scientific discovery have ushered in a new era of medicine, transforming our ability to treat, and in some cases cure, some of the most challenging diseases, including many cancers, rare diseases and autoimmune conditions. These advancements are due to the productivity of the United States' biomedical R&D ecosystem, which is sustained by a policy framework that is designed to support and advance America's leadership in the innovation of new medicines.

This year, America's biopharmaceutical companies relied on decades of infectious disease, vaccine, and manufacturing expertise to research and develop solutions against COVID-19 for patients in record time. The unprecedented collaboration among biopharmaceutical companies, government agencies and others in the public and private sectors has been the key to the global fight against COVID-19. This collaborative ecosystem is among our country's greatest strengths in moving medical advances forward and is one reason why the United States remains the global leader in biopharmaceutical innovation. Today, biopharmaceutical companies continue to invest in critical R&D and manufacturing scale-up to address the current pandemic, while also ensuring the continuity of other ongoing R&D efforts against other unmet patient needs.

The R&D Process for New Drugs Is Lengthy and Costly, With High Risk of Failure

From drug discovery through FDA approval, developing a new medicine takes, on average, 10 to 15 years and costs $2.6 billion.* Less than 12% of the candidate medicines that make it into Phase I clinical trials are approved by the FDA.



Key: IND=Investigational new drug application, NDA=New drug application, BLA=Biologics license application

*The average R&D cost required to bring a new FDA-approved medicine to patients is estimated to be $2.6 billion over the past decade (in 2013 dollars), including the cost of the many potential medicines that do not make it through to FDA approval.

The Complex Biopharmaceutical R&D Process

The R&D process typically begins with the screening of an enormous number of potential medicines followed by pre-clinical research and several phases of clinical trials which can take many years even under ideal circumstances. On average it takes 10 to 15 years for a medicine to make its way from the start of the R&D process to approval by the U.S. Food and Drug Administration (FDA). And only 12% of investigational medicines entering phase one clinical trials are ultimately approved by the FDA.[3]

The average cost to develop a new medicine is estimated at $2.6 billion, which includes the cost of medicines that fail, as most candidates never make it past a phase one clinical trial.[4] Rapid scientific and technical advances, alongside increasing regulatory burdens, are resulting in more complex clinical trials.

Today, clinical trials generate three times the data collected 10 years ago and clinical protocols have become significantly more complex, largely in response to increased regulatory requirements, contributing to growing R&D costs and increasing the challenges related to patient enrollment and retention.[5]

The nature of conducting research in areas of high scientific complexity and regulatory uncertainty is that failure is inevitable. But the information gained from these so-called "failures" help inform research and development projects and the knowledge base to advance the biopharmaceutical research enterprise. A recent analysis of nine different cancers found between 1998 and 2020, there have been 1,366 unsuccessful investigational drugs and only 115 gaining FDA approval.[6] Despite these challenges, today there are over 1,300 medicines in development to treat a wide range of cancers.[7]

America's biopharmaceutical research companies remain committed to bringing important new treatment options to patients. In fact, PhRMA member companies having invested more than a trillion dollars in R&D over the past two decades.[8]

The Evolving R&D Process

Researchers are constantly refining and improving the R&D process in response to new scientific and technological advances and evolving regulatory requirements. Increasing acceptance of innovative clinical trial designs, appropriate integration of the patient perspective and advancing the use of real-world evidence are speeding the development and regulatory review process and enhancing the competitive marketplace through the introduction of innovative new medicines.

The speed by which the biopharmaceutical industry was able to leverage its expertise and collaborate across the ecosystem to deliver safe and effective vaccines and treatment to address COVID-19 illustrate this ability to evolve and drive efficiencies in the R&D process. In achieving this feat, companies brought vaccine candidates into human clinical trials in a matter of months while simultaneously ramping up manufacturing capacity, including entering into manufacturing partnerships in the US and around the globe so that once a vaccine received authorization from the FDA, patients could swiftly access it.

Providing Hope to Patients

By all accounts, we are in an unprecedented period of medical discovery, driving the research and development of new cures and treatments for many of our most costly and debilitating diseases and providing new treatment options that allow many to live longer, healthier lives. However, in many ways, our work is just getting started. Realizing the promise and potential of the pipeline will require increased collaboration across a range of sectors and fields in order to harness novel scientific approaches, massive amounts of data, computational capabilities, and new technologies.

This dynamic innovation ecosystem has not occurred by happenstance; it is the result of a science-based regulatory framework and public policies that have formed an environment that fosters, incentivizes and enables innovative and collaborative efforts that give way to large-scale groundbreaking progress and biomedical advances. Our unique innovation ecosystem has made the United States a global bioscience leader and is built on policies that encourage public and private sectors to play complementary roles in drug discovery and development. To continue to advance medical discovery, public policies must continue to support this environment and avoid policies that could impede collaboration and progress through massive cuts to innovation investment or erosion of critical intellectual property protections. This will be critical to not only maintain U.S. global leadership in biomedical innovation, but to continue the fight against COVID-19 and ensure we are prepared for the next public health emergency.

1 PhRMA analysis of Adis R&D Insight Database.
2 G Long, Analysis Group, "The Biopharmaceutical Pipeline: Innovative Therapies in Clinical Development," July 2017.
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5 Rising Protocol design complexity is driving rapid growth in clinical trial data. TCSDD Impact Report. January 2021.
6 PhRMA.Researching Cancer Medicines: Setbacks and Stepping Stones. August 2020.
7 PhRMA. Medicines in Development for Cancer. December 2020.
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