THE DYNAMIC US 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 difficult, and the failure risks are higher. Right now, a new set of challenges to the R&D process is posed by the current pandemic. Despite these hurdles, research-based biopharmaceutical companies are committed to realizing the promise of the pipeline and are working with stakeholders across the dynamic U.S. R&D ecosystem 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 brighter.[1, 2]

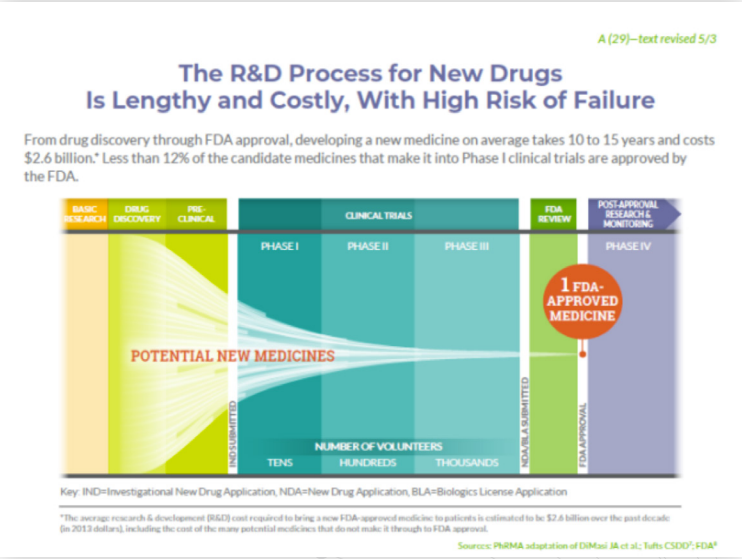
AMERICA'S BIOPHARMACEUTICAL COMPANIES PLAY CENTRAL ROLE IN R&D ECOSYSTEM

The biopharmaceutical industry's unique role in the research ecosystem is to utilize its scientific and industrial expertise to take the necessary risks to build on and further advance basic science research into safe and effective treatments that can be made available to patients. The complex ecosystem is marked by collaborations across industry, academic institutions, government agencies, venture capital firms, nonprofit foundations and others.

Today, as researchers are working tirelessly to combat COVID-19, PhRMA member companies are relying on this ecosystem to advance new treatments and are also collaborating with relevant global public health authorities – such as the FDA, NIH, CDC, the World Health Organization and many others across the world – to address the pandemic and ensure continuity of other development programs. This collaborative ecosystem is among our country's greatest strengths in moving medical advances forward and is one reason why the United States is the global leader in biopharmaceutical innovation.

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 FDA. And only 12% of investigational medicines entering 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. Rapid scientific and technical advances, alongside increasing regulatory burdens, are resulting in more complex clinical trials. For example, one study found that between 2001 and 2015, the total number of endpoints within a typical Phase III trial grew 86% and the number of procedures (including routine exams, blood work and x-rays) grew by 70%. Despite these growing challenges, biopharmaceutical research companies remain committed to bringing important new treatment options to patients. In fact, PhRMA member companies themselves have invested more than half a trillion dollars in R&D since 2000.[6]

WORKING TOGETHER TO BEAT COVID-19

In response to the COVID-19 pandemic, industry and regulatory authorities are applying novel approaches to the structure and conduct of clinical trials, facilities inspections, drug review, manufacturing and supply chain security to accelerate the development process and inform efficient regulatory decision-making. And while 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 FDA, companies have been working with regulators and amongst themselves to conduct critical processes in parallel and shorten timelines wherever safe and possible. For example, companies brought vaccine candidates into human clinical trials in a matter of months while simultaneously ramping up manufacturing capacity so that if a vaccine is approved, patients can swiftly obtain access.

One reason companies have been able to respond quickly during this crisis is due to scientists' familiarity with other strains of the coronavirus and longstanding investment in innovative platform technologies that could be brought to bear. During the pandemic, as they have with past public health emergencies, biopharmaceutical companies also look for pre-clinical compounds and medicines approved for other indications to see if they may have activity against the virus to speed the advancement of new treatment options to impact patients with COVID-19 as quickly as possible.

THE EVOLVING R&D PROCESS

Researchers are constantly seeking to refine 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.

While new scientific advances bring greater promise and complexity, the process is inherently fraught with a high degree of scientific and regulatory uncertainty, and there are often research setbacks. For example, between 1998 and 2017, 146 potential medicines for the treatment of Alzheimer's disease did not make it through clinical trials, with only four gaining FDA approval.[7] Despite these challenges, researchers remain committed to conquering challenging diseases such as Alzheimer's. Today, there are 92 Alzheimer medicines in clinical development or awaiting FDA review.[8]

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 and convergence across a range of sectors and fields to harness novel scientific approaches, massive amounts of data and computational capabilities and new technologies.

It is critical that we have a policy and regulatory environment that promotes innovation to fulfill the promise that these scientific opportunities represent for patients. To continue to advance medical discovery, we need to ensure a well-functioning, science-based regulatory system that keeps pace with the latest advances and ensures the timely review, approval and introduction of new medicines. This will be critical not only to improve the lives of patients, but also to maintain U.S. global leadership in biomedical innovation and to combat COVID-19.

[1] PhRMA analysis of Adis Database.

[2] G Long, Analysis Group, "The Biopharmaceutical Pipeline: Innovative Therapies in Clinical Development," July 2017.

[3] JA DiMasi, HG Grabowski, RW Hansen. Innovation in the pharmaceutical industry: New estimates of R&D costs. J Health Econ. 2016;47:20-33.

[4] JA DiMasi , Grabowski, RW Hansen. Innovation in the pharmaceutical industry: New estimates of R&D costs. J Health Econ. 2016;47:20-33.

[5] KA Getz, RA Campo. New benchmarks characterizing growth in protocol design complexity. Therapeutic Innovation & Regul Sci. 2018;52(1):22-28.

[6] PhRMA annual membership survey. Washington, DC: PhRMA.

[7] PhRMA. Researching Alzheimer's Disease: Setbacks and Stepping Stones. Fall 2018.

[8] PhRMA. Medicines in Development for Neurological Disorders. April 2018.