The Dynamic U.S. Research and Development Ecosystem

Our rapidly growing understanding of the underlying mechanisms of disease are fueling the development of new treatments and cures for patients. At the same time, the scientific and regulatory challenges associated with the drug development process have created more complexities and increased the risks associated with conducting research and development (R&D). The impressive progress that has been made in COVID-19 therapeutics and vaccines reflects just one example of the tireless efforts of America's biopharmaceutical research companies to apply their expertise to overcome the many challenges associated with the R&D process. The industry remains committed to working with partners across the health care ecosystem to combat global health threats and meet the needs of patients. Looking forward, the U.S. R&D ecosystem continues to leverage new scientific and technological advances to bring innovative medicines to patients with a wide range of diseases and conditions. With more than 8,000 medicines in clinical development globally–of which nearly 70% have the potential to be first-in-class treatments–the future has never been more promising.[1,2]

America's Biopharmaceutical Companies are Central to the R&D Ecosystem

America's biopharmaceutical companies are at the heart of the robust biomedical 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 and the immense investments the biopharmaceutical industry has made to advance progress for patients. In fact, PhRMA member companies have invested more than a trillion dollars in R&D over the past two decades in the pursuit of this endeavor, including more than $102 billion in 2021 alone, the highest level of investment on record.[3]

This collaborative ecosystem is among our country's greatest strengths in moving medical advances forward and is one reason why the United States has been the global leader in biopharmaceutical innovation. It is also why biopharmaceutical companies, government agencies and others across the public and private sector were so successful in collaborating in an unprecedented fashion in the global fight against COVID-19.

The Complex Biopharmaceutical R&D Process

The R&D process typically begins with the screening of an enormous number of potential medicines with the potential to address a disease, followed by pre-clinical research and several phases of clinical trials which can take many years. On average it takes 10 to 15 years and $2.6 billion 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).[4] And only 12% of investigational medicines entering phase one clinical trials are ultimately approved by the FDA.[5]

Today, rapid scientific and technical advances often result in more complex clinical trials and increased R&D costs. Clinical trials are generating three times the data collected ten years ago and trial protocols have become significantly more complex. In fact, the number of procedures required to complete a study, the number of endpoints measured, and the number of sites involved in a clinical trial have all increased dramatically in recent years.[6]

At the same time, researchers are constantly refining and improving the R&D process in response to new scientific and technological advances and evolving regulatory requirements. For example, the use of innovative clinical designs enables researchers to enroll fewer patients and finish trials sooner than those using traditional protocols while maintaining the same rigorous standards. Additionally, the use of decentralized clinical trial methods has been shown to significantly improve the efficiency of clinical trial recruitment and conduct.[7] Expanded use of real-world evidence is also contributing to a more efficient development and regulatory review process.

While failure is an inevitable consequence of conducting research in areas of high scientific complexity, researchers leverage the information gained from these failures to inform R&D projects and the collective evidence to advance the new treatments for patients. For example, one analysis of nine different cancers found between 1998 and 2020, there have been 1,366 unsuccessful investigational drugs, with just 115 gaining FDA approval.[8] And yet, despite the incredible uncertainty and risks involved in the R&D process, today there are over 1,300 medicines in development to treat a wide range of cancers.[9]

The biopharmaceutical industry's ability to evolve and drive efficiencies in the R&D and manufacturing process is perhaps most clearly illustrated by the speed by which the industry was able to leverage its expertise and collaborate across the ecosystem to deliver safe and effective vaccines and treatments to combat COVID-19. This effort was supported by Intellectual property (IP) protections which facilitated the countless collaborations that enabled companies to bring vaccine candidates into human clinical trials in a matter of months. The industry did this while also simultaneously ramping up manufacturing capacity and 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. Fortunately, the lessons learned over the past couple of years will have positive implications lasting long beyond our fight with COVID-19.

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. This dynamic innovation ecosystem has not occurred by happenstance; it has been the result of a 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.

But today, U.S. global leadership in biomedical innovation and patient access to lifesaving treatments is being threatened by a new drug price-setting law that upends the incentives that have been built into our competitive ecosystem. The misguided policies included in the law are projected to cause an immediate decline in R&D spending, resulting in fewer new medicines coming to market in the future.[10] By some estimates, these policies could sacrifice more than 100 new treatments over the next 2 decades.[11]

At the same time, policies that seriously undermine IP protections, such as those that waive international commitments to honor IP rights related to COVID-19 vaccines and treatments under the World Trade Organization (WTO) Agreement on Trade-Related Aspects of Intellectual Property Rights (TRIPS), are threatening U.S. global leadership in R&D and innovation. Compounding these threats are proposals to use march-in provisions of the Bayh-Dole Act to regulate drug prices. These march-in proposals not only fail to recognize the intent of the law–which is to ensure that federally funded inventions are incorporated into useful products – but they threaten to discourage that innovation in the process.

To continue to advance medical discovery, public policies should support the environment that has proven such a success in bringing forth the tremendous progress that we have seen in the development of groundbreaking medicines in recent years. That means an environment that is supported by a science-based regulatory system, strong IP rights, and a recognition that price-setting policies jeopardize both the engine of American innovation and our nation's preparedness for the next the next public health emergency.

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