UNLEASHING THE NEXT GENERATION OF **BIOTECHNOLOGY INNOVATION**

Modern biotechnology is a young industry. But in just over four decades, the scientists, researchers and entrepreneurs working in this field have firmly established themselves at the forefront of medical innovation.

The innovations and research coming from these enterprises have led to the successful development of new cures and therapies that are transforming the way we treat patients for a wide range of once-devastating diseases, including:

- **Hepatitis C** – a once incurable disease that now has cure rates above 90 percent[1];
- **HIV/AIDS** has gone from a death sentence to a chronic manageable condition[2];
- **More than 730,000 children's lives in the U.S. have been saved** in the last 20 years because of advances in vaccines[3];
- **The cancer death rate has fallen by 20% since its peak in 1991**, in large part due to medicines.[4]

Punching Above Their Weight

Biotechnology's strong track record can be traced directly to the men and women working in the field. For them, biotechnology is not just an occupation, it is a mission and a calling to solve the greatest challenges of our time: To unlock the essence of life itself and to use that knowledge in the service of compassion and in the name of hope. They are entrepreneurial. They are risk takers. They are driven by science and are stubborn in their refusal to accept the status quo.

Their passion and perseverance is why nearly 70 percent of the industry's clinical pipeline is attributed to small companies.[5] These companies also are on the cutting-edge of the next generation of innovation, including major advances in gene therapy, immunotherapy and RNAi therapy. These innovations are poised to transform medicine in the 21st Century.

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The vast majority of the companies working on these innovations, and across biotechnology, are small, pre-revenue enterprises. Their success in getting new cures and therapies across the finish line rests on one key factor: the ability to attract the enormous amounts of private capital required to fund these challenging and incredibly risky endeavors. This ability, in turn, depends on a public policy environment that supports innovation and incentivizes such investment, including continued advancement of scientific understanding; strong intellectual property (IP) rights and a reliable system for IP transfer, licensing, and collaboration; an efficient and predictable regulatory review process; and transparent payment systems that reward innovation and encourage free market competition.

Set-backs across any of these areas can cause the entire innovation ecosystem to falter, but the challenge can be particularly acute when it comes to capital formation. Private investment can flow to and shift among many different sectors, and investors have shown that they will flee areas like biotechnology when they think policy decisions could adversely impact an already risky investment.

Biotechnology Business Model

Biotechnology entrepreneurs take great financial risk to attempt to innovate and find cures and therapies for diseases -- and only occasionally do they succeed. When they do, patients benefit and investors receive a return on the investments made, allowing a continuing cycle of innovation. The reality of the market is that the return on successful medical innovation must be sufficient to provide a favorable risk profile for investors (VC or corporate) to continue to invest in the next generation of medical breakthroughs.

The biopharmaceutical industry is unique in that there are regulated pathways that allow for legal copies of drugs or therapies, known as generics or biosimilars, to be brought to market by competitors without having to do the same safety and efficacy studies that the innovator had to do. To give innovators a reasonable opportunity to secure a favorable return on their massive investments, federal law limits generic or biosimilar competition for a limited period of time.

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Those protections involve both patents and data exclusivity, which serve different and complementary purposes. Typically, by the time a drug is finally approved by the FDA, there is roughly only 10 years left of the original 20-year patent term. Patents protect the drug, and often its use or method of manufacture, and incentivize the R&D necessary to go from basic discovery, to translational and applied research, to the development and approval of an actual FDA-approved medicine that has been proven safe and effective through lengthy and expensive clinical trials. Data exclusivity, on the other hand, protects innovators against competitors "free riding" on all that innovative R&D by seeking FDA approval of identical or highly similar molecules based on the innovators' data.

In other words, patents protect the invention, while data exclusivity protects the R&D needed to turn that invention into an FDA-approved medicine. While patents are a critical form of intellectual property, they are not always sufficient. The combination of patents and data exclusivity, which run concurrently, allow an innovator a reasonably predictable period of time on the market before government-facilitated generic entry occurs.

During this time, however, innovative drugs often face vigorous brand-to-brand competition. As the Federal Trade Commission itself has noted, "[a]pproval of a breakthrough or pioneer drug product is increasingly followed by entry of a subsequent branded product(s). The head start that the breakthrough product has had over subsequent branded products has decreased over the past three decades from 8.2 years during the 1970s to 2.25 years in the 1990s." In fact, all of the breakthrough products studied during the 1990s had branded competitors in clinical development at or before their approval.[6] Thus the notion that there is a lack of market competition or monopolization by brand companies is misguided.

Once exclusivity and patents are exhausted, generics can enter the marketplace, society is rewarded with lower-priced medicines, and entrepreneurs continue the high-risk investment mode searching for the next medical breakthrough.

The system works well, benefitting patients with cycles of new innovations and lower-priced medicines over time. Today, approximately 88% of prescriptions are filled with generics.[7] Yet we have maintained a system that continues to incentivize the next medical breakthrough.

These breakthroughs not only benefit patients, but also drive value across the entire health care system by, for example, reducing the cost of hospital and nursing home expenses. Even the non-partisan Congressional Budget Office (CBO), which usually eschews any form of dynamic scoring, has officially embraced this concept in its financial projection methodology, utilizing a formula that credits each dollar of additional spending on medicines with a 20 cent reduction in other healthcare expenses.

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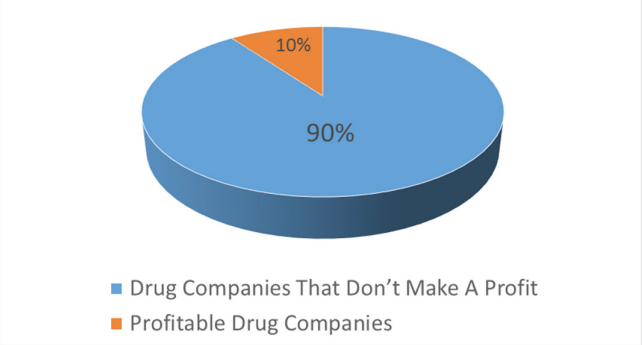
As one venture capitalist specializing in biotechnology recently wrote, "We waste trillions every year in a reactive health system, tending to the dying in hospitals and on untreatable family members languishing in long-term care. **Innovative drugs that cure diseases are not the enemy, they are the solution.**"

90% OF BIOPHARMA COMPANIES DO NOT EARN A PROFIT

Given the enormous amounts of capital and time required to bring a drug through the development and approval process, the fact is that, **of the approximately 1,200 biopharma companies in the United States, more than 90 percent of these enterprises do not earn a profit and focus on innovation R&D for future products**.[8]

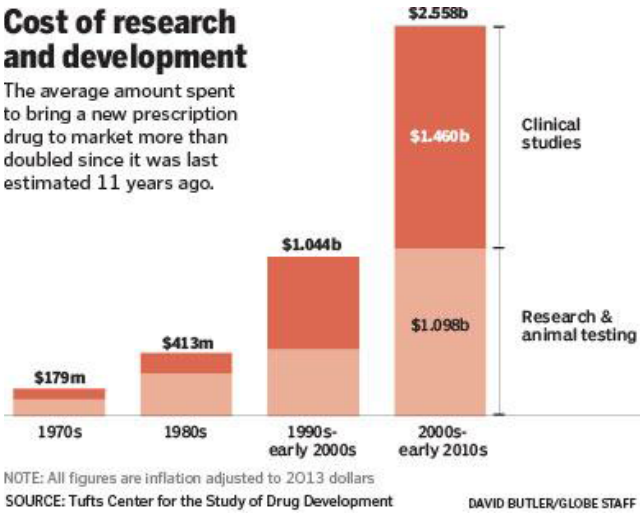


That's because **only five in 5,000 potential medicines that enter preclinical testing make it to human testing and only one of those five is approved for sale.**[9]

Compounding that, only two of every 10 drugs on the market ever earn back enough money to match the costs of R&D and the FDA approval process before their patent expires.

Also, the duration of the clinical phase of approvals for biopharmaceuticals has steadily increased, from an average of 4.6 years in 1990-1994, to an average of 7.1 years in 2005-2009. This has significantly increased the price of drug development.[10]

That's why bringing a new drug to market costs an average of nearly $2.6 billion, when you take into account the cost of failures, and why it can take more than 10 years to get a new drug approved.[11]



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Investors have to commit these large amounts of capital over this entire timeframe.

There simply is nothing like it in any other industry, and it is why public policy must be carefully calibrated to ensure the preservation of incentives to undertake this incredibly valuable but risky work.

Cost of Research

To finance their work, biopharmaceutical companies have committed more than $500 billion in the search for new medicines over the last

15 years. Last year, research investment totaled $51 billion, up from $15 billion in 1995. That means that biopharma spends five times more on R&D investment than the aerospace sector, and two-and-a-half times more than the software and computer industry.[12]

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Market Pricing

Discussion about the cost of innovative therapies that focuses solely or mainly on the list price belies the realities of the health care system today.[13]

Manufacturers provide billions of dollars in rebates and discounts on their innovative therapies annually to Federal, State and private payors, in addition to offering direct assistance through patient assistance programs.[14] These rebates occur in a number of ways. In the commercial insurance market, rebates and discounts are the result of market-based negotiations between manufacturers, insurers and pharmacy benefit managers. These discounts vary but can result in significant discounts as much as 50 percent[15] or greater depending on the program.

When the government is the payor, the vast majority of purchases have mandated rebates and discounts. For example, to participate in the Medicaid program, manufacturers are required to provide at least a 23.1% rebate on branded drugs and biologicals used by Medicaid beneficiaries.[16] In fact, these rebate requirements expanded under the Affordable Care Act.

Additionally, within the Medicaid program, states negotiate supplemental rebate agreements with manufacturers that result in further discounts on top of federal requirements. Other federal programs, such as those for active duty military and veterans or certain safety net providers, also receive significant discounts off the list price of biopharmaceuticals. Further, these programs capture additional discounts when the price of a branded biopharmaceutical increases more than the rate of inflation – a feature that does not exist for generic medicines.[17]

Discounts vary but can result in significant discounts as high as 50 percent or more.

In the Medicare program, discounted prescription drug prices are reflected in two ways. First, in the traditional, fee-for-service Medicare Part B program, the federal government reimburses providers for prescription drugs based on a formula that takes into account all of the rebates and discounts available in the commercial marketplace. In this way, the Medicare program also is able to benefit from the savings brought about by market-based contracting.

Second, in the case of Medicare Part D and Medicare Advantage (i.e., Medicare Part C), private insurers—that are responsible for administering these programs—negotiate rebates and discounts with manufacturers in much the same way as happens in the commercial insurance market.

The discounts resulting from these negotiations benefit the Medicare program in the form of lower annual plan bids, which Medicare uses to determine how much it will reimburse these insurers for covering participating Medicare beneficiaries (i.e., lower plan bids result in lower Medicare expenditures for these programs).

Patients also receive further direct assistance from manufacturers through the Medicare Part D Coverage Gap Discount Program.

Whether mandated, negotiated or voluntarily offered to patients, discounts are widely achieved in the majority of all drug sales in the United States. As a result, focusing on the list price of a biopharmaceutical ignores the realities of the health care market; it is like focusing on the MSRP when buying a new car – it is the starting point for a complex series of negotiations and discounts that ultimately lower the actual price significantly.

Long-Term Economic Benefits

In addition to the societal benefits of biotechnology for patients and the reduction in overall costs to our healthcare system, there also are clear benefits for the U.S. economy's long-term growth. According to the Department of Commerce, innovation has been responsible for two-thirds of economic growth since World War II. Medical innovation has been at the forefront of that growth, and biotechnology is poised to be a significant economic engine for the U.S. economy in the 21st Century.

In the U.S. today, there are more than 1.6 million men and women working in the biosciences. Over the past decade, the industry has added nearly 111,000 new, high-paying jobs.

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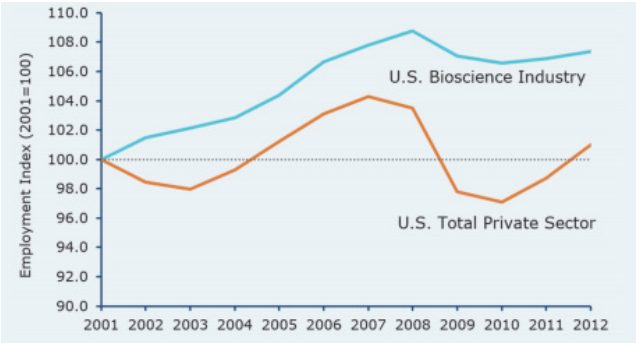

Economic output of the bioscience industry has expanded significantly with 17 percent growth for the biosciences since 2007, nearly twice the national private sector nominal output growth. And the industry continues its tradition of creating high-wage, family-sustaining jobs with average wages 80 percent greater than the overall private sector and growing at a faster rate.[18]

The U.S. leads the world in biotechnology and medical innovation. Take the countries that negotiated the Trans-Pacific Partnership (TPP). As has been reported, of the approximately 5,600 drugs in the pipeline among TPP nations, around 3,400 are being developed by U.S. companies.[19] This is not an accident. We have the scientific expertise. We have an investor base willing to take extraordinary risk on the next generation of innovation. And we have a carefully crafted policy infrastructure that helps support and sustain the innovation ecosystem.

Investing and supporting the pillars of successful biotechnology innovation will provide tangible long-term benefits for our families and loved ones in the form of new cures and therapies for a range of diseases, as well as good paying jobs that support and sustain strong communities.

ENDNOTES

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