Testimony of Tom DiLenge, President, Advocacy, Law & Public Policy
Biotechnology Innovation Organization
Before the U.S. House of Representatives Energy and Commerce Committee
Subcommittee on Health Hearing on "Examining the Drug Supply Chain"
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Mr. Chairman, Ranking Member Green, and Members of the Subcommittee: BIO appreciates the opportunity to speak with you today about the innovative biopharmaceutical development and delivery system and its enormous contributions to patient health and the U.S. economy.

My name is Tom DiLenge and I am the President for Advocacy, Law and Public Policy at the Biotechnology Innovation Organization, or BIO. BIO is the world's largest trade association representing biotechnology companies, academic institutions, state biotechnology centers, and related organizations across the United States and in more than 30 other nations. BIO is a unique association because it represents an entire technology, rather than a particular industry or sector. Our members operate at the intersection of biology and technology to discover innovative ways to not only heal the world, but also to help feed and fuel our planet in more sustainable and environmentally friendly ways.

BIO also represents the entire biotech innovative ecosystem – from universities and research institutes, to start-up biotechnology companies, to the private investors that risk massive amounts of capital to fund these companies, to the larger, established companies that play a critical role in bringing these amazing innovations through the development and approval process and into the marketplace.

Of our approximately 1,000 members, the vast majority of them are small companies engaged in some of the most challenging, cutting-edge research in the world. They typically have no marketed products and no profits, and thus are heavily reliant on private capital to fund their work. They take enormous risks every day to develop the next generation of biomedical breakthroughs for the millions of patients suffering from diseases for which there currently are no effective cures or treatments.

BIO is enormously proud of their entrepreneurial and innovative spirit, and the dedication of all of our members to alleviating human suffering. I am honored to testify before you on their behalf today to explain what it takes to sustain this dynamic ecosystem and to bring new cures through the incredibly risky development and regulatory approval processes to the patients who need them.

America's Role in Biomedical Innovation

Let me emphasize at the outset a fundamental fact about biomedical innovation: American biopharmaceutical innovation delivers more new drugs than the rest of the world combined. Almost 60 percent of all new medicines that treat patients across the world are innovated right here in the United States.[1]

This dominant American leadership is made possible by a number of factors:

- Outstanding scientists;
- Savvy entrepreneurs;
- World-class research universities;
- A culture of learning from setbacks;
- And investors willing to take risky bets on life-saving cures.

Yet other countries have similar capabilities, but don't drive innovation to the degree that America does. Why does America stand out and lead the world? Because we have a public policy environment that incentivizes investment in innovation. These policies include:

- Strong support for continued advancement and funding of scientific understanding;

[1] DeVol, R., Bedroussian, A, Yeo, B, "The Global Biomedical Industry: Preserving U.S. Leadership," Milken Institute, 2011, at p. 5.

- Strong and predictable intellectual property (IP) rights and a reliable system for IP transfer, licensing and collaboration;
- An efficient and predictable regulatory environment that strives to keep up with advances in biomedical science; and
- Payment systems that reward innovation and encourage free-market competition.

Yes, the American public has always had a love-hate relationship with drug companies, dating back to the late 1800s. But fortunately, even in the face of public pressure, Congress has steadfastly remained focused on curing disease and has opted against rash policy actions, including a refusal to impose artificial limits on the rewards for successful innovation.

Congress' strong, bipartisan support for the National Institutes of Health (NIH) biomedical research enterprise, which BIO has consistently endorsed, has been a critical component of our nation's success. Yet while NIH funds the basic academic research that often leads to breakthrough discoveries about our biology and genetics, it is the private sector that drives the applied R&D and approval of actual new medicines based on those new scientific understandings.

In fact, the biopharmaceutical industry's collective annual research budget is more than $150 billion – roughly five times the entire NIH budget – and our investments are growing much more rapidly than NIH funding.[2] About half of this private sector research – $75 billion a year – is invested right here in the United States, far more than any other industry in America.[3] In any given year, biopharmaceutical companies spend five times more on R&D than the aerospace industry – and more than double that of the

[2] See https://report.nih.gov/fundingfacts/fundingfacts.aspx; World Preview 2017, Outlook to 2022, EvaluatePharma, 2017, at p. 19; Factset, BIO Industry Analysis.
[3] Factset, BIO Industry Analysis.

software industry.[4] Indeed, our industry plows more of our revenues back into research and development than any other American industry – period – roughly one out of every five dollars of revenue we generate.[5]

These investments are paying off in big ways not only for patients living with terrible diseases and conditions, but also for American workers and our economy. Today, more than 1.7 million Americans are employed in the biosciences across the United States, and we are growing jobs at a rate double the national average.[6] These are good-paying, highly skilled jobs, and support another seven million American jobs throughout the economy.[7]

Biotechnology: A Young Industry with Enormous Public Health Contributions

Modern biotechnology is a young industry. But in just a few decades, the entrepreneurs, scientists, researchers and investors working in this field have firmly established themselves at the forefront of medical innovation. Indeed, 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 what they learn to treat and cure disease. These individuals are entrepreneurial. They are risk takers. They are driven by science and are stubborn in their refusal to accept human suffering or the status quo. So, let's pause to examine the miracles our young industry already has made possible.

[4] Pitts, P. J., Why people don't trust drug makers. *New York Post* (September 24, 2015). Retrieved from https://nypost.com/2015/09/24/why-people-dont-trust-drug-makers/
[5] Factset, BIO Industry Analysis.
[6] TEConomy/BIO, The Value of Bioscience Innovation in Growing Jobs and Improving Quality of Life, 2016. Retrieved from https://www.bio.org/value-bioscience-innovation-growing-jobs-and-improving-quality-life-2016
[7] Ibid.

Thirty years ago, the HIV/AIDS epidemic pushed our country and our healthcare system to the brink. Facing more than one million people infected – and nearly 100,000 deaths – American biopharmaceutical companies went to work, supported by an active patient community who demanded more flexibility from federal regulators.

By 1996, with the development and FDA approval of the first wave of antiretroviral drugs, the HIV epidemic abated. Death rates have plummeted by nearly 85% since 1995.[8] Since then, nearly a million premature HIV/AIDS deaths have been prevented, saving our economy $1.4 trillion dollars.[9]

What was once thought to be untreatable is now beatable. Today, a 20-year-old diagnosed with HIV can expect to live into their early 70s – a life expectancy comparable to that of a person without the disease.[10]

The biotech story doesn't end there.

Medical breakthroughs are delivering meaningful, measurable results for millions of patients facing a wide variety of serious diseases. For example:

- There has been a 22% decrease in the cancer death rate since 1991, resulting in millions of lives saved and an estimated $2 trillion in economic savings;[11]
- Today, 83% of children with cancer survive, compared to just 58% in the mid-1970s;[12]

[8] U.S Department of Health and Services, Centers for Disease Control, "Health, United States, 2013, With Special Feature on Prescription Drugs," 2014.
[9] Philipson T, Jena AB. "Who Benefits from New Medical Technologies? Estimates of Consumer and Producer Surpluses for HIV/AIDS Drugs. Forum for Health Economics and Policy." 2006;9 (2) Biomedical Research and the Economy, Article 3.
[10] 1 Samji H, Cescon A, Hogg RS, Modur SP, Althoff KN, et al., Closing the Gap: Increases in Life Expectancy among Treated HIV-Positive Individuals in the United States and Canada. PLoS ONE 8(12): e81355 (2013).
[11] Lakdawala DN, et al. An economic evaluation of the war on cancer. Journal of Health Economics. May 2010. 29(3):333-346.
[12] See https://www.bio.org/sites/default/files/BIO_Cancer%20Medicine.pdf

- Today, 80% of people with Chronic Myeloid Leukemia experience 10-year survival rates, compared to only 20% a decade ago;[13]
- Heart disease death rates decreased by nearly 30% between 2001 and 2010 alone;[14]
- In a single year in America, statins that lower cholesterol save 40,000 lives, prevent 60,000 heart attacks, and avert 22,000 strokes;[15] and
- New therapies are delivering more than a 90% cure rate for Hepatitis C, saving our health care system billions in reduced hospital costs, liver cancer treatments, and liver transplants.[16]

Innovation truly saves – not just lives, but real dollars as well. In fact, if biopharmaceutical researchers are able to develop a new medicine that delays the onset of Alzheimer's disease by just five years, America would save $367 billion in healthcare services by 2050.[17] To meaningfully bend the troubling healthcare cost curve, increased investment in new medicines is essential.

The Next Wave of Biomedical Advances Are Here

This is an extraordinary time for biotechnology. The therapies in development and coming to the market are unlike any we've seen in the history of medicine. Our companies are making discoveries that were unimaginable a decade ago. We truly are in a new era of medicine, and the science and its promise for alleviating human suffering is galloping forward.

[13] Journal of Managed Care, Nov. 2012
[14] American Heart Association, *Annual Statistical Update Report,* December 2014. Retrieved from http://newsroom.heart.org/news/new-statistical-update-looks-at-worldwide-heart-stroke-health
[15] Health Affairs, at content.healthaffairs.org/content/31/10/2276.abstract
[16] Kabiri M, Jazwinski AB, Roberts MS, Schaefer AJ, Chatwal J. "The changing burden of hepatitis C virus infection in the United States: Model-based predictions." Annals of Internal Medicine. 2014;161 (3):170-180.
[17] Alzheimer's Association, *Changing the Trajectory of Alzheimer's Disease: How a Treatment by 2025 Saves Lives and Dollars*. Retrieved from https://alz.org/documents_custom/ALZ_Trajectory_InfoSheet_01.26.15_Final.pdf

The days of traditional chemical drugs that treat broad classes of patients in blunt ways are giving way to the development of entirely new ways to treat and ultimately cure disease for targeted patient populations using living organisms, including a patient's own cells.

These include advances such as immuno-oncology – in which we attack cancer by activating the body's own immune system against it, while leaving healthy cells alone. And cellular and gene therapy – in which we use a patient's own cells to develop a medicine tailored for that patient, or use other genetic techniques to repair or replace defectives genes causing disease. We've already seen the first wave of these advances reach the marketplace, with many more already in the FDA regulatory process, including novel medicines that utilize these techniques to treat childhood leukemia and blindness.

Overall, our industry has nearly 6,000 new medicines in development for a wide range of life-threatening or debilitating diseases, with 74% of these clinical projects targeting new mechanisms of action – so called "first in class" medicines that work in entirely new ways to treat and cure disease.[18]

This is innovation at its best. The only thing that can stop our march forward is bad public policy.

The Biopharmaceutical Ecosystem by the Numbers

As *The Economist* magazine has noted, "Creating new drugs through biotechnology is at the risky end of a business in which superhuman stamina and bottomless pockets are minimum requirements."[19] Thus, to

[18] Emerging Therapeutic Company Investment and Deal Trends 2007-2016, BIO Industry Analysis, 2017, at bio.org/iareports; Long, G. The Biopharmaceutical Pipeline: Innovative Therapies in Clinical Development, Analysis Group, 2017; The Analysis Group, *The Biopharmaceutical Pipeline: Innovative Therapies in Clinical Development*. July 2017. Retrieved from: http://phrma-docs.phrma.org/files/dmfile/Biopharmaceutical-Pipeline-Full-Report.pdf

[19] Biotechnology: Fever Rising. *The Economist* (Feb. 14, 2014). Retrieved from: https://www.economist.com/news/business/21596557-there-are-reasons-hope-latest-biotech-boom-will-not-be-followed-another

understand the biopharmaceutical innovation ecosystem and its pricing dynamics, you have to understand the numbers. Here are the key ones:

- **70%:** that's the percentage of innovative clinical programs that are being led by small companies, which rely heavily on venture capitalists, angel investors, or partnerships with larger pharmaceutical companies to provide the enormous amounts of private capital required to fund these challenging and incredibly risky endeavors.[20]
- **90%:** that's the percentage of clinical programs that ultimately fail to lead to an FDA approval; in fact, the success rate of clinical trials can be even less than that, particularly in areas like Alzheimer's and cancer.[21]
- **92%:** that's the percentage of biopharmaceutical companies that are unprofitable at any given time.[22]
- **10-15 Years:** that's the time it takes on average to secure FDA approval of a new medicine, from initial discovery of a potential new molecule or approach, through pre-clinical and clinical programs, and through the FDA regulatory and approval processes.[23]
- **$2.6 Billion:** that's the average cost to develop and secure approval of a new medicine, taking into account all the trial and error and research failures along the way, and the cost of capital; this figure has skyrocketed in recent years, doubling since just 2003.[24]
- **36th:** that is where the biopharmaceutical industry ranks among domestic industries in terms of return on investment, despite the popular media narrative of excessive drug industry profits.[25]

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[20] Emerging Therapeutic Company Investment and Deal Trends 2007-2016, BIO Industry Analysis, 2017, at bio.org/iareports.
[21] Clinical Development Success Rates 2006-2015, BIO Industry Analysis, 2016, at bio.org/iareports; for example, since 1998, 123 medicines in development for Alzheimer's have not made it through clinical trials, while only 4 have been approved – resulting in a 97% failure rate. See PhRMA, *Researching Alzheimer's Medicines: Setbacks and Stepping Stones*, Summer 2015. Retrieved from http://phrma-docs.phrma.org/sites/default/files/pdf/alzheimersetbacksreportfinal912.pdf

[22] Factset, BIO Industry Analysis.
[23] DiMasi J., Grabowski, H., Hansen, R. Innovation in the Pharmaceutical Industry: New estimates of R&D Costs. Journal of Health Economics, 2016, at pp. 20-33.
[24] Ibid.
[25] Factset, BIO Industry Analysis.

- **89%:** that's the percentage of all prescriptions in America that are for lower-cost generic copies of once-branded pharmaceuticals.[26]

The hundreds of billions of dollars needed to support this private biopharmaceutical ecosystem each year come from two basic sources: private and public investors, and sales revenues from existing marketed medicines. Thus, it is the revenues from the 10% of successful clinical programs, and the 11% of prescriptions for branded medicines, that have to reward investors and support the continuing R&D innovation cycle. And they must do that before generics can copy them and enter the market, because once generics enter the market, prices and innovator market share fall dramatically. Notably, no other component of healthcare (e.g., hospital or physician fees) experiences such reductions in price over time – a fact that is often ignored during debates regarding drug pricing.

As noted above, the costs of drug innovation have increased dramatically over the past 15 years. This is partly due to increased regulatory and payer demands for more and better data on drug safety and effectiveness. But it also has much to do with the new era of genomic and personalized medicine. We are tackling the most challenging of problems, and the more we learn about our biology and the basis of disease, the more complex our R&D processes become. Furthermore, while these costs go up and up, the patient populations to be served are becoming more and more targeted, reducing the ability to spread these costs across wide patient populations.

Thus, this critically important innovation ecosystem can continue to flourish only if it has the confidence and financial backing of investors. Biotech investors – like all investors – expect a reasonable return on their investments, free from artificial, government-imposed restrictions or erroneous assumptions about

[26] Association for Accessible Medicines. *Generic Drug Access & Savings in the U.S.* (2016). Retrieved from https://accessiblemeds.org/sites/default/files/2017-07/2017-AAM-Access-Savings-Report-2017-web2.pdf

investment returns.

So, let me be absolutely clear here: investment in the biotech sector is directly influenced by the public policy debate in Congress and the states, and by the policy positions taken by our leading public officials. We have seen that impact over and over again over the decades, with the latest example occurring just last year amid all the negative focus on drug prices during the 2016 presidential campaign. While the stock index for market-stage drug companies declined slightly during 2016 (only 3%), the index representing small, research-stage biotech companies plummeted 38% over that same time.[27] Here are some more troubling data points from 2016:

- The number of new biotech IPOs dropped in half;
- The dollars spent of acquisitions of small biotech companies dropped by 43%;
- The dollars invested by venture capital in late-stage financing rounds dropped by 33%; and
- The number of biotech licensing deals dropped by 17% and the value of upfront payments to small biotech companies as a result of such deals was cut in half.[28]

Indeed, these smaller companies – the ones that account for 70% of the cutting-edge clinical programs underway today – are the proverbial canaries in the coal mine. When the public and policy debates turn against innovation, these small companies are the first to feel the negative results – making it harder for them to raise the capital needed to advance their R&D programs. And for patients waiting for the next modern biotech miracle, that is really bad news.

[27] Based on index performance in 2016 for the LifeSci Biotechnology Clinical Trials Index and the LifeSci Biotechnology Products Index. See Life Sci Index Partners, at http://www.bioshares.com; Factset, BIO Industry Analysis.

[28] Emerging Therapeutic Company Investment and Deal Trends 2007-2016, BIO Industry Analysis, 2017, at bio.org/iareports.

Yes, in the biotech sector, risk is an everyday reality. Indeed, biotech is synonymous with risk. But political and policy risk is something different. It can cripple innovation.

The Right Way to Enhance Patient Access and Affordability of New Drugs

BIO and our members recognize that too many patients, even those with insurance, cannot afford access to the life-saving cures and treatments that biotech companies are developing. We stand with the Members of this Committee, the Congress, and the Trump Administration in our shared commitment to addressing this serious problem.

But to accomplish this, we have to harness – not abandon – the free market that has delivered amazing innovations for patients and made America first in the world in biomedical innovation. BIO supports enhancing drug affordability through competition. If we act smartly to promote market-based reforms that spur greater competition and efficiencies in our healthcare sector, we can improve patient access to the innovations of today, while preserving incentives to discover the next generation of innovations for the patients of tomorrow.

That's why BIO has joined with stakeholders across the healthcare spectrum – including insurers, PBMs, employers, and patient groups – in a coalition that developed and supports consensus, market-based reforms to lower drug costs without harming innovation. Through the Council for Affordable Health Coverage,[29] BIO and our allies are working to:

- Increase marketplace competition by speeding regulatory approval of more innovative drugs, and promoting greater and faster generic and biosimilar entry once patents and exclusivities for innovator drugs have expired;

[29] https://www.cahc.net/prescription-drugs/

- Move towards a drug payment system that is based on value and patient outcomes rather than volume, by removing regulatory and legal barriers that hamper value-based arrangements and communications between innovators and payers;
- Empower patients and providers with more data on drug costs and value to help them make more informed choices; and
- Oppose innovation-killing ideas like price controls, drug importation, or direct government "negotiation" of drug prices in Medicare.

The Council estimates that, if fully adopted and implemented, these reforms could lower national health expenditures by $71 billion a year.[30] This approach also is consistent with the landmark 21st Century Cures Act shepherded through Congress last year by this Committee on a strongly bipartisan basis, as well as this Committee's more recently enacted Food & Drug Administration Reauthorization Act (FDARA). BIO was a strong supporter of both of these bills, which we believe will help expedite the delivery of new innovations to patients in need, while also speeding competition among branded medicines and from more generics and biosimilars. We all want to see FDA approve generic drugs as efficiently as possible and for the backlog of generic drug applications to be reduced quickly. More choice and competition is good for patients and the healthcare system overall.

That said, it should be noted that, generally speaking, the United States has a robustly competitive market for drugs, where innovators compete vigorously with one another to produce safer and more effective medicines within the same class, and then compete on price as part of negotiations with powerful, sophisticated, and aggressive commercial middlemen such as insurance companies and pharmacy benefit managers who control patient access to these innovative products. While there are pockets of exceptions to this competitive environment, the reality is that the average innovator drug has a short period of time

[30] Ibid.

on the market without competition from other similar products (roughly two years[31]), and nearly nine out of every 10 prescriptions filled in America are for cheaper generic copies of once-branded drugs.[32]

Conclusion

America is one of the only countries left in the world that doesn't treat a vibrant, risk-taking biotech industry like a regulated public utility – because we know that doing so will cause investors to flee this critical sector and put their money elsewhere. And this much we also know: If we adopt price restrictions favored in countries with socialized medicine, we can expect to get the level of innovation found in countries with socialized medicine.[33]

BIO recognizes that it's sometimes easy to think of biotech companies as simply manufacturers of expensive pills and biologics. But nothing could be further from the truth. Whether we are suffering from disease or caring for a loved one who is, the biomedical innovation ecosystem gives every one of us the most precious gifts of all – hope that tomorrow will be better than today, and more time to share with those who mean the most to us.

We're life savers.

We're the "cure" to the growing societal cost of disease.

And we're job creators, investors, and drivers of American economic growth and global technological leadership.

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[31] See "Nearly All Later Entrants to Drug Classes Were in Clinical Testing or Regulatory Review Before First-in-Class Approval," Tufts Center for the Study of Drug Development, November 3, 2015, at http://csdd.tufts.edu/news/complete_story/pr_ir_november_december_2015

[32] Association for Accessible Medicines. *Generic Drug Access & Savings in the U.S.* (2016). Retrieved from https://accessiblemeds.org/sites/default/files/2017-07/2017-AAM-Access-Savings-Report-2017-web2.pdf

[33] Joseph H. Golec and John A. Vernon, "Financial Effects of Pharmaceutical Price Regulation on R&D Spending by EU versus US Firms," Pharmacoeconomics 28, no. 8, (2010): 615–628.

With Congress' continued support, we will put more people to work, lower health care costs, and heal the world.

Thank you for the opportunity to present our views today. For more information on drug costs and value, I would encourage you to visit www.drugcostfacts.org, a comprehensive website that answers many of the questions patients and policymakers frequently ask about the drug supply system by reference to expert studies and other independent data sources.

And I stand ready to answer any questions you may have during my testimony as well.