



James C. Greenwood
President & CEO

October 22, 2019

Dear President Trump:

On behalf of the Biotechnology Innovation Organization (BIO) and our 1,000 members representing a broad cross-section of the biotechnology ecosystem, I am pleased to present our views in response to the White House Office of Science & Technology Policy's request for information on the bioeconomy.

As outlined in the attached submission, U.S. leadership in biotechnology has made a huge contribution to the health of people around the world and to the health of our common planet. And its potential to accelerate transformative, positive change in the future is unmatched by any other sector of our economy. We urge the Administration to seize the opportunity to expand on this American leadership, by taking action and supporting the pro-innovation policies outlined in this submission.

We look forward to our continued partnership with the Administration and other stakeholders in this critical endeavor.

Most Sincerely Yours,

A handwritten signature in black ink that reads "Jim Greenwood". The signature is fluid and cursive, with the first name "Jim" being more prominent than the last name "Greenwood".

James C. Greenwood
President and CEO

BIOTECHNOLOGY'S ROLE IN FEEDING, FUELING AND HEALING THE WORLD AND PROMOTING AMERICAN GLOBAL LEADERSHIP

The Biotechnology Innovation Organization (BIO), representing 1,000 members across the biotech ecosystem, is organized around a central mission: to advance public policy that supports a wide range of companies and academic research centers working to apply biology and technology in the food, agriculture, health, manufacturing, and energy sectors to improve the lives of people and the health of the planet. BIO represents the entire U.S. bioeconomy, and we welcome the opportunity to partner with the U.S. government to promote our sector's growth for the benefit of the American economy and society as a whole.

The state of our bio-based economy is strong, yet we still have tremendous potential for growth. Biotechnology companies directly employ 1.74 million people in more than 85,000 business establishments across the United States. U.S. bioscience companies have increased employment by 273,000 jobs since 2001, with net job gains recorded by the industry in all but two of the last 15 years. Bioscience industry wages are consistently higher, on average, than those for the overall economy. In fact, the average U.S. bioscience worker earned nearly \$99,000 in 2016 – 85 percent greater than the average for the overall private sector. The bioscience industry's economic impact on the U.S. economy, as measured by overall output, totaled \$2 trillion in 2016 alone.

Biotechnology is making an enormous difference in improving the health of our loved ones and our planet. Nearly one million premature HIV/AIDS deaths have been prevented, with an associated economic value of \$1.4 trillion. New therapies have delivered a 90 percent cure rate for Hepatitis C, saving billions in reduced hospital costs. [Routine childhood vaccinations developed by biotech companies prevented 732,000 early deaths](#) from 1994 to 2013. Biotech crops, such as those that require no tilling, saved 27 billion kg of carbon dioxide emissions. Biotech-enhanced farming systems saved 452 million acres of lands from plowing and cultivation and decreased use of pesticides by 8.4 percent since 1996. And since the passage of the Renewable Fuel Standard (RFS), the production of low-carbon, sustainable biofuels have reduced greenhouse gas (GHG) emissions in the transportation sector by 600 million metric tons (MMT), while the development of renewable chemicals and bio-based products removed 12 MMT of CO₂ from the manufacturing sector in 2016 alone.

Yet America's continued success and leadership are not guaranteed. Many countries have great scientists; what has set the U.S. biotech sector apart has been thoughtful, bipartisan public policy approaches that create a favorable climate in which to undertake the lengthy and risky job of investing in and developing the next biotech breakthroughs. Today, foreign countries are taking overt steps to streamline regulatory systems and speed pathways to market, often with direct government support as part of national bioeconomy strategies. America cannot rest on our laurels and must not take our global leadership for granted. The U.S. bio-based economy depends on preservation of those incentives that facilitate robust and steady private investments.

More specifically, the key drivers of successful growth in our bio-based economy are:

1. Robust funding of public- and private-sector scientific research
2. Strong and predictable intellectual property rights, with a flexible and well-functioning technology transfer system
3. A dynamic capital investment environment, with competitive returns on investment capital
4. Science- and risk-based regulation of biotechnology innovation that speeds the path to market and minimizes post-approval obstacles to commercialization and adoption
5. Market-based mechanisms for the sale of biotechnology products that promote patient and consumer access and continued incentives for innovation
6. Public support that embraces the positive influence of biotechnology

Because the United States has embraced these drivers of success, the U.S. biotech industry leads the world in bringing new breakthroughs to market. For example, U.S. companies innovate more new medicines than the

rest of the world combined (56 percent). We are incredibly successful in driving capital formation, despite the facts that only one in 10 drugs in the clinic make it to a successful Food and Drug Administration (FDA) approval and that 90 percent of biopharmaceutical companies fail to earn any profit at all. The reason for this success is simple. It's because ours is the closest thing to a free market left in the world when it comes to drug pricing – allowing a sufficient market-based return on the few new medicines that do earn FDA approval each year to subsidize the many failures and fuel the continuing research and development (R&D) cycle. This unique characteristic of the U.S market gives our Nation an enormous competitive advantage in attracting investment capital and leads to the impressive economic benefits described above.

BIO urges the Administration not to abandon or pull back on the policies that have made our bioeconomy the strongest and most dynamic in the world, but rather to seize the opportunity to reform our policies to meet the challenges posed by the next wave of biotechnological innovations.

CURRENT CONTRIBUTIONS AND FUTURE POTENTIAL: TRANSFORMATIVE THERAPIES, FOOD AND FARM INNOVATION, AND SUSTAINABLE FUELS AND BIO-BASED MANUFACTURING

a. Transformative Therapies for Patients

BIO's members are rapidly advancing a new wave of innovative, transformative therapies that provide a significant, durable benefit and value for patient health outcomes, delivery of care, and overall healthcare spending. These therapies are aimed at serious and rare diseases where patients often have limited treatment options today. Examples include cellular therapy, which refers to the use of cells to replace or repair tissue and/or other cells damaged by disease, or to attack cancer cells. Cell therapy may be used for a variety of diseases and conditions, including cancer, sickle cell disease, beta thalassemia, and HIV. Transformative approaches also include gene therapy – a type of medicine designed to treat a genetic disease by adding the functioning gene or genes into a specific cell (e.g., liver cells, bone marrow cells), which allows the patient's body to return to good health. Gene therapy also can be used to reduce the activity of a harmful gene. Currently, there are many gene therapies being developed to treat multiple diseases, including hemophilia, inherited retinal diseases, myeloma, phenylketonuria (PKU), and Huntington's disease. There already are three approved gene therapy products in the United States, including for congenital blindness and leukemia. BIO's members have 388 gene therapy programs under development. Transformative therapies like these have the potential to truly transform the treatment of serious and rare disease, and even cure diseases once thought to be incurable.

In addition, BIO's health sector members have a long history of success developing preventative public health tools such as vaccines that allow people to live healthier, longer, and more productive lives. Among children born between 1994 and 2018, the Center for Disease Control & Prevention (CDC) Vaccines for Children (VFC) program is estimated to save nearly \$1.9 trillion in societal costs, including \$406 billion in healthcare costs. We now can prevent more than 20 once-common diseases through vaccination. Ensuring continued innovation in vaccines and a robust immunization infrastructure (both at home and abroad) creates U.S. jobs across the fields of R&D, manufacturing, public health, and medicine. Innovation in vaccines also helps keep Americans healthy and at work, ensuring our economy is not being slowed by unnecessary costs or lowered productivity.

b. Biology-based Food and Farm Innovations

For over two decades, the products of agricultural biotechnology have been commercially available and widely used by a growing number of farmers around the world. In the United States, 90 percent of corn, cotton, canola, papaya, soybean, and sugar beet seeds planted contain at least one biotechnology-derived trait. Farmers use these products because they enable the production of more food and feed on fewer acres using reduced pesticide applications. Farmers are also growing higher valued consumer-oriented crops, such as non-browning apples

and potatoes that reduce food waste, and soybeans with a more heart healthy oil composition. Animals derived through biotechnology will soon be on the market and many other animal innovations are in the pipeline.

The research, development, and widespread commercialization of the current set of agricultural biotechnology products occurred as U.S. government agencies conducted pre-market regulatory oversight of these products under the auspices of the Coordinated Framework for Regulation of Biotechnology. The United States' science-based regulatory approach enabled technology developers to generate and commercialize many highly beneficial products, while assuring consumers and markets that such products have received appropriate pre-market scrutiny and are as safe and nutritious as their conventional counterparts.

Developers of innovative biology-based tools and products for our food supply and agricultural systems face many challenges, particularly given macro-level market trends. Those challenges, however, are matched by a tremendous opportunity for biology to pave the way for more sustainable and climate-resilient food and farm economies. BIO is the home of a diverse array of innovators using a variety of enabling biotechnologies to develop products for our sustainable food and farm future – for food and feed inputs; for crop and animal production and enhancement; and for human and animal health. This dynamic ecosystem has been bringing beneficial biotechnology products to market for years. As interest in sustainable, climate-resilient, and healthier food and farming grows among consumers and investors, this sector is set to grow if the policy and market access conditions will allow, enabling BIO's members to feed a growing world in ways that will advance human and animal health, reduce GHG emissions, eliminate the need to develop marginal lands for food production, and create sustainable ingredients that taste better and enhance nutritional health.

c. Sustainable Fuels and Bio-based Manufacturing

Biotechnology is enabling a dramatic paradigm shift in the production of fuels and chemicals. Using biological processes to convert renewable biomass and waste feedstocks into everyday products has created new markets for agricultural crops, crop residues, and waste streams. These technologies produce sustainable biofuels, renewable chemicals, and bio-based products that provide a cost-competitive alternative to fossil fuel-based products and that generate added value through job creation and environmental and public health benefits.

Whether it is low-carbon biofuels reducing GHG emissions; sustainable personal care products eliminating animal testing; or bio-based materials that reduce and eliminate plastic waste...our companies are on the frontlines of addressing the world's most pressing environmental challenges in ways that align with consumers' values and promote economic benefits that coincide with environmental improvement. Next-generation advanced and cellulosic biofuel facilities already are producing biofuels with near-zero, or even negative lifecycle GHG emissions. Policymakers can unleash growth opportunities in the bio-based economy by supporting policies that contribute to building these and other sustainable bio-based value chains.

PUBLIC POLICY IS KEY TO CONTINUED ADVANCEMENT OF THE U.S. BIOECONOMY

a. Emerging Biotechnology Companies Are Foundational to a Strong U.S. Bioeconomy

A key factor in understanding the bioeconomy is that, despite the intense public focus on so-called "Big Pharma," more than 90% of the biopharmaceutical industry is made up of small, emerging companies – R&D-intensive, with large operating losses and no marketed products, and heavily reliant on substantial amounts of private and public investment capital for a long period of time prior to any commercialization. In 2018, a record \$12.3 billion in venture funding went to U.S. emerging therapeutic companies, with 95% of this amount allocated toward novel R&D and only 5% into R&D for improvements to existing drugs. Venture investment into innovative U.S. therapeutic companies continues to outpace Europe (5.7x), Asia (4.6x), and the rest of the world (35x). Additionally, first-time Series A financing broke a record in the U.S. with 109 new companies receiving funding, indicating a robust interest in early-stage biotech. Further, U.S.-based R&D-stage emerging

therapeutic companies were able to raise \$5.1 billion from 47 IPOs in 2018, a record dollar amount and the 2nd highest number of IPOs in a decade. Public market follow-on offerings for U.S.-based R&D-stage emerging therapeutic companies remained strong, with \$11.5 billion raised in 2018 across 118 offerings.

Globally, the total active clinical-stage programs reached a record 6,984, with emerging companies accounting for 73% of these programs and U.S.-based companies accounting for more than half of the total. Emerging companies currently have 94 marketing applications for new drugs (NDA/BLAs) under review at the Food & Drug Administration (FDA), with U.S.-based companies accounting for 62% of these emerging company submissions. For more investment trend information, please see BIO's report at <http://go.bio.org/rs/490-EHZ-999/images/BIO%202019%20Emerging%20Company%20Trend%20Report.pdf>.

b. Support Robust Capital Markets and Tax Policies for Emerging Biotechnology Companies

Pre-revenue innovators undertaking the decades-long development process intrinsic to scientific advancement must raise vast sums of money to fund their research. The Jumpstart Our Business Startups (JOBS) Act has increased the flow of capital to innovative small businesses, while also decreasing capital diversions from the lab to unnecessary regulatory compliance burdens. In fact, in the five years after the JOBS Act was enacted in 2012, there were more than 212 biotechnology IPOs. The prior five years saw only 55 biotechnology companies go public. As of today, the number of biotech companies that have gone public since JOBS Act passage exceeds 330, with a surge in financing for early-stage biotechs. For example, in the five years after the JOBS Act passed, there were 48 IPOs by early-stage biotechs (pre-clinical R&D and Phase I clinical trials), compared to just three preclinical and Phase I IPOs in the five years before the JOBS Act. As of the end of July 2019, we have seen 92 early-stage companies go public.

Emerging biotech companies raised \$17 billion through JOBS Act IPOs – an average of \$80 million per offering during the law's first five years. These newly public innovators subsequently raised an additional \$16 billion through follow-on offerings. In addition to spurring the development of new medicines for millions of patients, the JOBS Act has helped build a stronger bioeconomy with JOBS Act biotech companies currently employing more than 48,000 employees, and with a collective market value of more than \$165 billion.

Efforts are underway to improve the JOBS Act for emerging companies by expanding eligibility for regulatory relief for pre-revenue companies via exemptions from the auditor attestation of internal controls over financial reporting requirement under Sarbanes-Oxley (SOX) 404(b). These efforts recognize that, because most biotechnology companies remain pre-revenue for a decade or more until they receive their first product approval, long past the original five-year exemption from SOX 404(b) granted by the JOBS Act, 404(b) can create a damaging diversion of capital from science to compliance. BIO published a report (https://www.bio.org/sites/default/files/BIO_EGC_White_Paper_02_11_2019_FINAL.pdf) demonstrating that SOX 404(b) compliance reduces market capitalizations, significantly increases audit fees, forces companies to exit public markets, and reduces innovation such as through R&D that results in fewer patents.

There are other legislative and regulatory efforts underway that would further enhance public capital formation and foster the continued growth of emerging public biotechnology companies. These include efforts to ensure that SEC oversight of proxy advisory firms foster accountability, transparency, responsiveness, and competition, and to enhance short-selling transparency in order to shine a light on manipulative trading behaviors that disincentivize long-term investment in innovation. The current practices of proxy firms and short-sellers have a particularly damaging impact on small biotechs due to our unique business model.

In addition, there are improvements to the tax code that would ensure that emerging, pre-revenue companies could better utilize venture and public investment dollars to build their companies and support research and development activities. For example, protection of investments in start-ups from inadvertently triggering the

net operating loss (NOL) limitations under Section 382 of the tax code would be a boon to small biotechs. R&D-intensive biotech start-ups typically rely on investor capital for more than a decade, through multiple financing rounds, before realizing any product revenue. As a result, they frequently accumulate substantial NOLs in these early, pre-revenue years. Fixing the unintended impact of the Section 382 limitations would permit capital-intensive start-ups to conduct multiple fundraising rounds without jeopardizing the value of their accumulated NOLs – a reform that will foster more investment, economic growth, job creation, and continued American leadership in biopharmaceutical and bio-based technology innovation. Additionally, expanding the ability of companies with no income tax liability to benefit from the R&D Tax Credit and reduce their payroll tax liability would be of tremendous benefit for small biotechnology companies.

It also is critical to prevent any further cuts to the Orphan Drug Tax Credit, which has been a foundational element of the Orphan Drug Act's success. There are approximately 7,000 rare diseases known to exist and 30 new ones identified each year, collectively impacting millions of patients who are depending on the development of treatments that don't yet exist for their diseases. Since the law's passage in 1983, hundreds of new therapies have been approved by the FDA to treat these rare diseases – compared to just 34 approvals before enactment. However, there is much more work that needs to be done to address the unmet medical needs of patient suffering from rare diseases. The majority of the research and development in rare diseases is being conducted by emerging companies, and any further erosion of this critical tax incentive could have a chilling effect on their ability to grow and support development of innovative rare disease treatments at the same pace we have seen for the past three decades. For more information, please see our report at <https://rarediseases.org/assets/files/white-papers/2015-06-17.nord-bio-ey-odtc.pdf>.

Finally, it is important that the new rules proposed by the Committee on Foreign Investment in the U.S. (CFIUS) to expand jurisdiction to review foreign investment in the United States for national security concerns is done in a manner that does not unduly disrupt foreign investment in the U.S. biotech industry. While confronting legitimate national security concerns is paramount, we do not want to undermine U.S. leadership as the key destination for biotechnology companies – which has been the bedrock of an innovation-based U.S. economy. BIO will continue to work with CFIUS to ensure both goals are obtainable. Please see: <https://www.bio.org/sites/default/files/Final%20BIO%20Comment%20Letter%20on%20CFIU%20Pilot%20-%2011.9.2018.pdf>.

c. Support Federal Funding of Basic Research and a Strong Public-Private Partnership in Biosciences

The federal government's long history of generously funding basic biomedical research is an important foundation for the Nation's bioeconomy. Today, even though the proposed 2020 National Institutes of Health (NIH) budget is about 20% below what it was 15 years ago (in today's dollars), the American public invests upwards of \$35 billion annually in basic bioscience. This sustained investment has created a rich infrastructure of foundational scientific knowledge that has led to opportunities for large private follow-on investments in applied biotech R&D. For example, it is estimated that around 30% of NIH-funded basic research later becomes relevant to applied new drug R&D, and that every public dollar that is spent on basic bioscience stimulates approximately 5-10 dollars in private R&D investment and real-world product development. America's life sciences companies invest upwards of \$100 billion annually in R&D, of which 85% occurs in the United States. This world-leading public and private investment is a critical ingredient of the broader U.S. bioeconomy.

A second critical ingredient is the technology transfer and commercialization laws and policies that were pioneered by the United States starting in the 1980s. The Bayh Dole Act and the Stevenson Wydler Act have played a catalytic role in stimulating domestic innovation across all sectors, and especially in the life sciences. Under these laws, clear rules govern the patenting and licensing of federally-funded inventions, so that private entities can undertake the risky, complex, and expensive follow-on R&D that is necessary to translate and

further advance basic research into real-world bioscience products. The federal government, academia, and biotech companies have thus long played complementary roles in the U.S. life-sciences innovation process.

Unfortunately, this highly successful innovation ecosystem is under threat from calls for heavy-handed government intervention. Proposals to exercise so-called government “march-in rights” to take away the rights of licensee companies after large R&D investments were made, or to establish government price limits for biotech products that might be developed based on federally-funded basic research, would be counterproductive to the shared goal of promoting innovation and devastating to future public-private partnerships. Biotechnology companies could not justify spending hundreds of millions of dollars developing a product if the government could step in 10 or more years later and either force a compulsorily license of their intellectual property to competitors or impose price controls on their marketed products – simply because at some earlier point in the discovery process, the government funded a small research grant relevant to the project. In this regard, it is worth noting that, while NIH grants may range from a few hundred thousand dollars to a few million, the private sector investment in a successful drug ranges from a few hundred million dollars to a billion or more.

A third component of U.S. success has been the NIH Small Business Innovative Research program (SBIR), which provides critical support for start-up biotechnology companies. This competitive grant program enables the best start-up companies in the United States to test concepts on early-stage clinical programs that have the most potential to benefit the public. Start-up companies successful in obtaining SBIR funding are in a better position to compete for private sector dollars to fully establish their businesses and advance their first-in-class clinical development programs. The NIH I-Corps is another beneficial program that funds mentoring and networking opportunities for new entrepreneurs seeking to commercialize promising biomedical technology.

Similarly, the successful adoption and deployment of biotechnologies in agriculture and manufacturing have been enabled by the U.S. Department of Agriculture (USDA) and the U.S. Department of Energy (DOE) research programs. USDA’s Agriculture and Food Research Initiative (AFRI) has been fundamental to advancing the agricultural sciences to improve rural economies and create new sources of energy. DOE’s Office of Energy Efficiency and Renewable Energy (EERE) invests in clean energy technologies that strengthen the economy, protect the environment, and reduce dependence on foreign oil. A total taxpayer investment of \$12 billion in EERE’s R&D portfolio has yielded more than \$388 billion in net economic benefits.

Lastly, it is important that the U.S. continue to support STEM education so that, as the bioeconomy grows, we have a domestic workforce that can take advantage of the increasing number of high-paying scientific jobs.

d. Protect and Promote U.S. Companies’ Intellectual Property and Regulatory Data Exclusivities

Private investment in biotechnological innovation is closely linked to the availability of intellectual property (IP) protections. Patents disclose and protect valuable inventions and provide a means for licensing, partnering, and technology transfer that is critical especially for smaller biotech businesses. Trade secrets protect manufacturing know-how, research leads, and similar competitive information. And for some types of drugs, biologics, and crop protection products, regulatory data exclusivities provide a limited period during which copy-cats cannot engage in free-riding on the innovator’s R&D investment. Together, these forms of IP enable biotechnology businesses to secure the large financial resources needed to advance biotechnology products to the marketplace, and to engage in the partnering and technology transfer that is necessary to translate basic scientific discoveries into real-world solutions for disease, pollution, and hunger.

Our Nation’s commitment to robust IP laws has coincided with the development of a domestic biotechnology industry that is without equal in the rest of the world. IP laws are about the “long game,” where the effect of today’s policies will become apparent only a decade from now, just like the new therapies or agricultural products that enter the market are the result of IP laws and incentives that were in place 10-15 years ago.

Thus, it is critical that we avoid myopically focusing on current complaints from certain quarters about the IP system without appreciating the system's longer-term benefits to society. Calls to reduce regulatory data exclusivity for biologic drugs, or to legislatively limit the enforceability of biotech patents, should be viewed with great skepticism if the goal is to stimulate long-term private investment in potentially transformative biotechnological advances. The U.S. bioeconomy will be best served by a stable legislative and regulatory climate that provides business certainty, long-term predictability, and a level international playing field.

To these ends, the data exclusivity and patent dispute resolution provisions governing biologics and biosimilars should be maintained in their current form, and the Administration should work with foreign governments to provide U.S. biotech companies the same level of IP protections in these foreign markets that the United States is already providing to foreign companies in our own domestic market. The successes and high research productivity of U.S. biotechnology enterprises has not been lost on foreign governments, including China, who are trying to kick-start their own bioeconomies in an effort to catch up to, or even leapfrog, the United States. Instances of forced technology transfer demands or disclosure of trade secrets as a condition of market access or regulatory approval, or special patentability rules that disadvantage biotech innovators, are all being deployed as tools by some foreign governments to create unfair advantages for their own nascent bioeconomies. If the United States is to maintain its global leadership in biotechnology and ensure that U.S. biotech companies can fairly compete internationally, such practices should be proactively and aggressively countered.

At the same time, certain destabilizing developments in our own domestic patent system deserve the Administration's attention. Over the past eight years, the law of patent-eligible subject matter under Section 101 of the Patent Act has undergone great change in ways that has created growing business and investment uncertainty, placed the U.S. system at a competitive disadvantage with foreign systems, and triggered calls for help from the U.S. Patent & Trademark Office (USPTO), the courts, and many elements of the business community. Likewise, the new system of administrative patent validity trials bears watching, as concerns over procedural fairness and repetitive attacks on valuable patents have not been fully addressed by recent improvements made by the USPTO. On both issues, the Administration should support stabilizing legislation.

e. Maintain FDA's Global Regulatory Leadership as Science and Technology Evolve

To maintain the U.S. position as the global leader in biotechnology, we must continue to be the gold-standard for regulatory approval and oversight. Today, the FDA leads the world in science-based regulation due to its flexible approaches and expedited pathways for breakthrough innovations. But the FDA must be able to attract and retain the world-class personnel and resources necessary to review the next-generation of complex and ground-breaking medicines such as cell and gene therapies. The FDA also must focus intently on advancing and enabling modern approaches to drug development, such as patient-focused drug development, validation of novel biomarkers and endpoints, modernization of clinical trials, utilization of real-world evidence, and modernization of manufacturing technologies. Continued modernization of development and review processes will improve how patients are able to participate in clinical trials and how companies can demonstrate and communicate the value of therapies to patients, and will create more effective and efficient processes for biotechnology companies to meet the FDA gold-standard of approval in a timely manner.

The 21st Century Cures Act and the FDA Reauthorization Act of 2017 went a long way in helping to achieve these goals. However, as we look to the growing pipeline of complex biotechnology products that have the potential to fundamentally change how we treat disease, it is imperative that we continue to provide the FDA with the staff, expertise, tools, and resources to continue to be the best regulatory agency in the world. Not only will this help enable timely access to ground-breaking treatments for the millions of U.S. families suffering from disease, it will help reduce health care costs, promote greater competition in biopharmaceutical markets, and incentivize biotechnology companies to grow their businesses here in the United States. For additional

information on this topic, see <https://docs.house.gov/meetings/IF/IF14/20170322/105752/HHRG-115-IF14-Wstate-HolcombeK-20170322.PDF>; https://www.bio.org/sites/default/files/Cures_Support_Letter_House.pdf.

f. Preserve Market-Based Reimbursement and Reform Payment Systems for Transformative Therapies

BIO's member companies are committed to developing truly transformative treatments and cures for patients with serious, unmet medical needs. At the same time, we 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 are equally committed 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. 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.

This includes increasing marketplace competition by promoting greater generic and biosimilar entry once patents and exclusivities for innovator drugs expire; moving towards a drug payment system based on value and patient outcomes rather than volume; empowering patients and providers with more data on drug costs and value to help them make more informed choices; and opposing innovation-killing ideas like price controls, foreign reference pricing, drug importation, or direct government “negotiation” of drug prices. We urge the Administration to work with BIO, patients, and stakeholders across the healthcare sector to adopt holistic, patient-centered solutions to drug affordability and access, and to abandon ideas that would undermine the U.S. bioeconomy and harm patients by importing foreign socialized medicine price controls – as the Administration is purportedly considering. It is worth emphasizing that the adoption of such price controls in foreign markets such as Europe has eviscerated their once-thriving biopharmaceutical investment and innovation economies.

As we work to increase patient access to innovative therapies, the Administration must do more to modernize the healthcare system to meet this goal. The new wave of transformative therapies is providing a significant, durable value for patient health outcomes, delivery of care, and overall healthcare spending. Examples of transformative therapies include cellular and gene therapies, which are truly personalized medicines. These innovative drugs and biologics have the potential to truly transform the treatment of serious and rare diseases, but patient access will remain at risk until reimbursement pathways catch up to this fast pace of innovation.

The reimbursement framework, particularly in public programs such as Medicare and Medicaid, should prioritize delivery of these breakthrough medicines as they bring the promise of positive benefits for overall healthcare spending and patient health outcomes. BIO is committed to working in partnership with the Administration and with other stakeholders to develop innovative payment models and financing approaches to ensure patient access to these novel therapies. But the Administration also needs to take urgent regulatory action to remove existing barriers to such modernized payment approaches for these next-generation therapies.

Each therapy in the transformative medicines space also will have individualized applications and require unique clinical circumstances. Patients will be treated across varying healthcare settings with differing associated care needs by specialized providers. The reimbursement system should provide appropriate reimbursement for these therapies and ensure that healthcare providers have the ability to provide these groundbreaking therapies through adequate reimbursement for the associated care they deliver.

g. Promote Greater Public Health, while Advancing Our National Security

Biotechnology capabilities and technologies aid our national preparedness for naturally occurring public health threats, intentional bioterror events, and accidents involving biological agents. Biotechnology companies working to develop countermeasures against chemical, biological, radiological, and nuclear (CBRN), pandemic influenza, and emerging infectious diseases are thus helping to protect our national security, while also

contributing to our bioeconomy. The U.S. government infrastructure put in place following the September 11th and Anthrax attacks in 2001 have been highly successful, with the Biomedical Advanced Research and Development Authority (BARDA) recently reaching an inspiring milestone: the 50th FDA-approved product in just over 15 years after establishment of the agency. The new vaccines, antimicrobials, diagnostics, and treatments developed by companies in partnership with the U.S. government and stockpiled for our national preparedness have created many highly skilled jobs across the country. In addition, investments in novel platform technologies that leverage our growing understanding of DNA and our ability to sequence microorganisms are continuously working to lower the economic costs of development in this space. The application of genetic sequence data will allow for more rapid and accurate development of medical countermeasures for both known and unknown threats to our Nation that could cripple our economy.

Continued investment in biodefense and national health security is imperative to protecting our nation's health and our nation's economy. The societal cost of responding to a pandemic has consistently been estimated to be high. In a September 2019 report, the White House Council of Economic Advisors estimated that the economic damage to the United States of a pandemic could range from \$413 billion to \$3.79 trillion. Ensuring continued innovation in vaccines and a robust infrastructure (both at home and abroad) creates U.S. jobs across the fields of R&D, manufacturing, public health, and medicine. Innovation in vaccines also helps keep Americans healthy and at work, ensuring our economy is not being slowed by unnecessary costs or lowered productivity.

Antimicrobial resistance (AMR) is an urgent threat. A recent study estimates as many as 162,000 Americans are dying from resistant infection annually, placing AMR as the third leading cause of U.S. death. Antimicrobial medicines' ability to treat and prevent infections plays a critical role in enabling medical innovation, including cancer chemotherapy, transplantations, and complex surgeries. They also are central to our national security by helping us respond and prevent both direct resistant biothreats and secondary infections such as those seen with influenza pandemics. To keep pace with resistance, a robust pipeline of AMR products is needed, including antibiotics, antifungals, vaccines, and other new technologies.

Unfortunately, due to unique economic challenges for antimicrobials, investment in this space has declined in recent years, with several companies closing their doors and others on the brink of bankruptcy as they struggle to raise funding for development and survive commercially. A package of incentives is needed to both stabilize the pipeline and drive investment back into this area. The proposed Developing an Innovative Strategy for Antimicrobial Resistant Microorganisms (DISARM) Act aims to address reimbursement challenges for qualifying antimicrobials, while strengthening stewardship that ensures their appropriate use. This bill, if enacted by the Congress, would send a strong signal that the U.S. government is taking action to address AMR, and we urge the Administration to proactively support this legislation. To further drive investment to this space, a "market pull" incentive – one that rewards the successful approval of a novel antimicrobial product addressing an urgent public health threat – also is needed. This reward would create a certain return on investment and ensure we have the robust pipeline of antimicrobial medicines this Nation needs. To date, no nation has implemented a pull incentive, despite global stakeholder consensus on its impact. We recommend the Administration work with BIO and other stakeholders to consider how to implement a pull incentive as soon as possible. For more information on this topic, please see <https://www.whitehouse.gov/wp-content/uploads/2019/09/Mitigating-the-Impact-of-Pandemic-Influenza-through-Vaccine-Innovation.pdf>; <https://www.cdc.gov/vaccines/programs/vfc/protecting-children.html>; <http://www.cidrap.umn.edu/news-perspective/2019/02/new-estimates-aim-define-true-burden-superbug-infections>; and <https://www.who.int/news-room/fact-sheets/detail/antibiotic-resistance>

h. Speed New Food & Farm Innovations to Market

Over the past twenty years, biotechnology techniques have continued to develop and now include more targeted and precise tools around which there is significant agricultural research and development effort. BIO is excited about new innovations, like gene editing, that will transform food and farm systems and enable the creation of so many beneficial products for farmers and consumers. Advancing and facilitating the adoption of innovations and technology for agricultural production and sustainable rural development has been a key goal of this Administration, as recently reflected in the White House’s Executive Order on Modernizing the Regulatory Framework for Agricultural Biotechnology Products (E.O. 13874). A regulatory climate that fosters innovation will be an important component in meeting that goal and ensuring development of a set of precise yet flexible tools for meeting the agricultural and environmental challenges facing farmers and society more broadly.

Science- and risk-based regulations that are practical and workable are the key to harnessing the resources necessary to address these big challenges and to provide enhanced opportunities for economic growth and job creation. The United States has been and continues to be an innovation leader in food and farm biotechnology. But other countries are taking steps to outflank the United States with respect to gene editing. We urge the U.S. government to take the following regulatory steps to ensure strong U.S. leadership continues:

- The White House should ensure that biotechnology regulatory oversight across the Federal government is functional, predictable, legally defensible, risk proportionate, and science based.
- USDA should finalize proposed revisions to its agricultural biotechnology regulations (7 CFR Part 340) that appropriately reflect these new innovations.
- EPA should publish guidance or proposed regulation to clarify under what circumstances, if any, products of gene editing would be subject to FIFRA registration.
- FDA should publish guidance clarifying whether and how food and feed derived from gene-edited plants will be subject to its consultation process.
- FDA should abandon its current “new animal drug” approach to oversight of gene-altered animals, and work with stakeholders and other Federal agencies to develop a more workable regulatory approach.
- Relevant agencies should promote favorable regulation of fermentation-based food, flavors, proteins, feed, and ingredients.

At the same time, BIO recognizes that long-term innovation successes are driven by more than just sound regulatory policy. Public and marketplace support are critical as well. BIO is committed to driving authentic dialogues with stakeholders to identify shared values and energize public understanding about innovation in food and farming, and we welcome the U.S. government’s support for such efforts.

i. Promote Sustainable Biofuels and Bio-based Manufacturing

Over the past decade, supportive government policies unleashed industrial biotech innovations and fostered investment in the bio-based economy – creating high paying jobs in rural communities and a value-added market for commodity producers. For example, the RFS has made the United States a leader in the development of advanced and cellulosic biofuels, creating new end user markets for transportation fuels in aviation and heavy-duty transport. Unfortunately, uncertainty surrounding EPA’s implementation of the law is undermining investment and development of new technologies that could create a more robust sustainable fuels sector. We urge more concrete action from the Administration to address this concern.

Reliable tax incentives also are critical to the development of this still-nascent sustainable fuels industry. The year-to-year nature of tax incentive renewals for advanced and cellulosic biofuels makes it difficult for companies to take advantage of these tax incentives to move projects to commercial scale, by making it harder to attract investment and predictably reduce the cost of production.

More broadly, the USDA Rural Development Energy Programs have been integral to driving investment and development of renewable chemicals and bio-based products that are creating more sustainable manufacturing processes. Continued policy support for these technologies will position the United States as a leader in the development of 21st century “green” manufacturing.

As we seek to reduce carbon and other pollutants in our environment, the U.S. government needs to do more to promote R&D and adoption of carbon capture and utilization (CCUS) biotechnologies, which aim to capture waste carbon in the form of methane, carbon oxide emissions, or gasified wastes and convert it to renewable and low-carbon chemicals, bio-based products, and advanced biofuels. Encouraging and incentivizing farmers to improve the capture of soil carbon, reduce applied nitrogen fertilizer needs, and improve yield through application of beneficial soil microorganisms and other enhanced farming practices will promote a low-carbon economy and provide the sustainable feedstocks needed for bio-based products.

As with the other biotech sectors, long-term innovation success in industrial applications requires sound science and risk-based regulatory policies. The intensifying pace of biotech R&D, new developments in synthetic biology and gene editing, and recent experience in engineering commercial-scale industrial biotech processes augment projections for economic growth. But underpinning that growth is the ability to speed pathways to the market. We ask the Administration to promote broad regulatory acceptance of, and where applicable, regulatory preference for, innovative and sustainable biotechnologies. In order to accelerate new breakthroughs, we must regain momentum and reignite investment in the bio-based economy through more far-reaching and predictable policies. In particular, BIO urges the Administration to leverage its authority to establish a sustainability platform under Sections 5 and 8 of the Toxic Substances Control Act. Such a sustainability platform could be the basis for overdue reforms to remove artificial regulatory barriers that deny new bio-based industrial chemicals a level regulatory playing field with equivalent, petroleum-based competing products.

j. Use U.S. Trade Policy to Promote the U.S. Bioeconomy

An effective U.S. government trade policy is critically necessary to address tariff and non-tariff barriers that affects the trade of, and innovation in, biotech products globally. In particular, the U.S. bioeconomy needs a proactive trade agenda focused on enhancing IP protection abroad, a harmonized and science-based regulatory environment, fair and equitable technology transfer policies, and access and enforcement policies that appropriately value American innovation and are governed by the rule of law. We applaud that the United States is actively negotiating agreements with key trading partners such as China to address systemic trade practices such as forced technology transfer and IP theft that threatens biotechnology ecosystem across sectors.

With respect to biopharmaceutical products, U.S. leadership in multilateral fora and bilateral trade agreements is essential to ensure trading partners adopt and respect core incentives for biotechnology innovation. BIO welcomes progress made in recent U.S. bilateral trade agreements, in particular with Mexico, Canada, and Korea, to require greater transparency and accountability of foreign government market access policies governing innovative biopharmaceuticals. BIO believes that modern trade agreements should commit U.S. trading partners to provide non-discriminatory access to new medicines and shoulder a fair share of the costs of innovation. Such provisions should include clear timetables for pricing and reimbursement decisions, clear justifications given for government decisions, the right to appeal decisions to an independent body, and provisions that ensure fair reward for innovative products within national healthcare systems. A thriving biotech industry will require that sound valuation and financing decisions are made especially with respect to new, potentially curative technologies, such as gene and cell therapies and gene-editing techniques.

U.S. leadership also must continue to bring trading partners up to U.S. intellectual property standards and to combat unfair practices such as forced technology transfer and intellectual property theft. BIO strongly believes the U.S. standard of data protection for biologics (12 years) should be the basis for all trade negotiations. And

more broadly, the U.S. government needs to develop an IP promotion strategy that recognizes the unique needs relating to these new biotechnologies, which may by their nature be harder to protect against unfair exploitation by copycats and other competitors, especially in foreign countries that do not fully respect American IP rights.

With respect to agricultural biotechnology, U.S. leadership is essential to ensure that U.S. agriculture can benefit from advances in science that reduce its environmental footprint while improving crop production. Many U.S. trading partners, including China and Europe, maintain unjustified, non-science barriers that delay the approval of new biotech products. To reduce the potential for trade disruption, biotech companies will often delay commercialization of new products in the United States until China and Europe have approved the same products. Such delays impact U.S. competitiveness and cost our economy dearly. A recent study estimates Chinese delays between 2011 and 2016 reduced farm income by \$8 billion and U.S. GDP by \$11 billion.

For new innovations like gene editing, the global regulatory landscape is unclear, and there is a risk that genome-edited products will be brought under outdated, discriminatory, and highly burdensome regulatory frameworks previously adopted for transgenic ag-biotech products, even though these newer products of gene editing do not contain foreign DNA. This creates the potential for enormous barriers to entry for this emerging industry, potentially limiting the use of this game-changing technology to only the biggest companies and in only large-scale crops. The United States currently is working with like-minded governments to chart a more reasonable path forward for new innovations in precision biotechnology. The U.S. government also has joined many governments from across the Americas, Africa, and Asia to support agricultural applications of precision biotechnology. This international effort is a clear signal to the world that innovations in precision biotechnology, like genome editing, should not face arbitrary and unjustified treatment by regulatory authorities. We applaud these efforts and encourage renewed urgency in their implementation.

k. Ensure Unrestricted Access to Biological Data and Resources

In a number of international fora, some countries are demanding that use of genomic data, often referred to as digital sequence information (DSI), give rise to financial and other benefit-sharing obligations on the part of those who develop products utilizing such data. If accepted, these financial demands could materially diminish the incentives for BIO's members to invest in the use of biological materials to which such expansive demands by foreign governments could apply. In addition, international institutions and even some U.S. states are adopting or exploring restrictions on access to and transfer of genomic data or other biological resources (such as pathogens). These efforts could restrict the ability of U.S. companies to conduct R&D efforts critical to public health and the growth of the bioeconomy. BIO encourages the Administration to enhance its engagement to protect against the adoption of such ill-informed and counterproductive policies.

CONCLUSION

BIO and our member companies stand at the forefront of the U.S. bioeconomy and we are proud that we have helped to make the United States the global leader in biotechnology innovation. We urge the Administration to seize the opportunity to expand on this American leadership, by taking action and supporting the pro-innovation policies outlined in this submission. We look forward to our continued partnership in this critical endeavor.