Life Sciences Pennsylvania – Federal Advocacy Priorities for 2022

Pennsylvania is a global leader in researching and developing new therapies, cures, and devices that help patients live longer, healthier lives. This innovation is due in part to the policy and regulatory ecosystem we enjoy in the United States.

Life Sciences PA works annually with its Federal Policy Committee and national partners to develop a set of policy issues and recommendations that will support and enhance the country's life sciences ecosystem, and maintains Pennsylvania's leadership in life sciences innovation.

Education on the Value of Innovation

Successfully healing patients or providing them enhanced quality of life drive the passion behind innovation. However, the process by which new medicines and technologies are created is time- and resource-intensive. Studies have shown it takes more than 10 years and $2 billion to move an innovative new drug through the research-and-development and clinical trial process to approval.

This process is fraught with failure as fewer than 12 percent of medicines entering clinical trials are ultimately approved by the U.S. Food and Drug Administration. This failure is one of the reasons biopharmaceutical companies need to recoup some of those costs through products that make it to the market. Recouping those dollars allows companies to pursue further basic, translational, clinical and manufacturing research needed to turn basic science into a safe, usable and effective therapy for patients.

In fact, the biopharmaceutical industry regularly invests more of its revenues in R&D than any other industry. As a recent Congressional Budget Office report highlights, for many years the number averaged 18-20 percent, but in recent years that number has exceeded 25 percent.

Life Sciences PA works to ensure the Pennsylvania Congressional Delegation, comprised of two senators and eighteen members of the House of Representatives, understands this process and that patient access to groundbreaking therapies and cures through appropriate reimbursement in our market-based system are responsible for the United States' leadership in biopharmaceutical innovation.

Supporting Policies to Improve Public Health

As we slowly emerge from the global pandemic it is important to remind legislators of the critical role life sciences organizations had in development and manufacturing diagnostics, therapeutics and vaccines to combat COVID-19. Our member entities worked collaboratively and tirelessly to address the pandemic, and that work continues today as our members research and develop medicines and technologies for future pandemics and public health crises.

Life Sciences PA supports policies, like the *PASTEUR Act* and the *DISARM Act*, that help support and incentivize the development of novel antibiotics to combat antimicrobial resistance (AMR), a growing public health challenge that, unabated, could result in 10 million deaths per year by 2050.

Supply Chain Challenges

Many sectors of the U.S. economy continue to struggle with development and manufacturing supply chain issues as industries recover from the global pandemic. The life sciences industry is no different, but it faces many unique challenges. Those challenges include finding raw materials, obtaining component materials from manufacturers, computer chip shortages, and fulfilling workforce needs, all while needing to ensure these products are safe, sterile, and coming from thoroughly vetted suppliers. These challenges persist as many of our member organizations are at the forefront of developing vaccines, diagnostics, and therapeutics for COVID-19 and are looking ahead to other public health challenges.

Life Sciences PA recognizes the need for public policies that ensure supply chains are sufficiently prepared to respond to future pandemics and public health crises. As legislation moves forward to address these challenges Life Sciences PA supports:

- Robust funding of the Biomedical Advanced Research and Development Authority (BARDA), the Strategic National Stockpile (SNS) and other relevant departments at the Office of the Assistant Secretary for Preparedness and Response (ASPR) to accelerate the discovery and production of biomedical countermeasures and technologies.
- Flexibility at the FDA to appropriately and efficiently respond to crises so that facilities can be inspected and approved in a timely fashion and critical products can safely obtain Emergency Use Authorizations where appropriate.
- Increased coordination between public and private entities to ensure strategies are in place to effectively respond to increased demand in the face of supply chain shortages and a workforce that is ready, willing, and able to meet those needs.

Improving Patient Access

Patients provide critical testimony on the effect of policy and regulations in their treatment and quality of life. Remaining focused on the voice of the patient is at the core of all innovation in the life sciences. Especially as medicines have become more personalized, the role of the patient in researching and developing new medicines has become increasingly important. Life Sciences PA works with our more than 50 patient advocacy group members to amplify the voice of the ultimate beneficiary of our member company research and development, the patients.

Some of the policies that affect patient access include:

<u>Preserving Medicare Part D</u>
Medicare Part D was created as part of the Medicare Modernization Act of 2003, and is an optional program to help Medicare beneficiaries pay for self-administered prescription medicines. In 2018 approximately 43 million (72 percent) of the 60 million Medicare beneficiaries utilize a Medicare Part D plan. The success of Medicare Part D, which continues to cost less than Congressional Budget Office projections and enjoys an almost 90% satisfactory rating among beneficiaries, is due largely to the private-sector competition and robust negotiation the program utilizes. Additionally, it has ensured

largely unfettered access to groundbreaking therapies and cures for beneficiaries facing debilitating and deadly diseases.

Life Sciences PA has serious concerns about proposals, like striking the non-interference clause, that could ultimately hurt a patient's ability to access novel medicines.

Protecting Medicare Part B

Medicare Parts A and B, sometimes referred to as "original Medicare," provide coverage for beneficiaries in the hospital/inpatient setting and outpatient services, respectively. In particular, Medicare Part B covers physician-administered prescription medicines, such as vaccinations, and injectable and infused drugs/biologics.

Life Sciences PA is opposed to proposals that disrupt access to biopharmaceuticals for patients in Medicare, such as indexing medicines to international price controls. Though not currently a threat, policies like this simply import prices from foreign countries where strict government-controlled prices limit patient access to the breakthrough medicines available to Americans. Additionally, policies like this can adversely affect patients in rural areas where cutting reimbursements to community providers could shift patients to more expensive, and farther away, care settings such as hospitals.

Insurance Benefit Design and Access Restrictions

It's important that when discussing patient access, we also highlight the drug pricing supply chain in the United States. While much attention is focused on the cost of prescription medicines, which account for approximately 10-14% of nationwide health care costs – a number that's remained consistent for decades – the real issue is the out-of-pocket costs borne by patients.

The cost of medicines is the cause of much debate by the public and elected officials and determining who pays for what medicines and at what cost is an important discussion to have. Life Sciences PA believes all parts – pharmacy benefit managers (PBMs), health insurance companies, pharmacies, healthcare providers, and manufacturers – of our complex drug pricing system have a role to play in this debate. It is incumbent upon all of us to ensure all parts of the prescription drug supply chain are acting in good faith and working to keep costs, especially those out-of-pocket costs borne by the patient, at an accessible level.

Life Sciences PA is committed to educating legislators on how insurance benefit design and various actors in the drug pricing supply chain can affect the out-of-pockets costs patients pay at the pharmacy. As noted above, the drug pricing, payment and distribution system is complex and includes many stakeholders. Life Sciences PA believe that all those stakeholders must be included in any and all discussions around lowering the cost of prescription medicines for patients.

Additionally, Life Sciences PA has significant concerns about insurance policies, such as step therapy (sometimes referred to as "fail first"), prior authorization, and co-pay accumulator policies that can delay or deny patient access to new medicines.

Importation of Prescription Drugs

Life Sciences PA is opposed to proposals that allow for the importation of prescription medicines. Though a common idea to lower the price of prescription drugs, policies that promote this idea could severely undermine the safe, closed drug-supply chain we enjoy in the United States. Several past FDA commissioners, from both Democrat and Republican administrations, have warned of the potential consequences of allowing drug importation, and the Congressional Budget Office has found that importation will do almost nothing to actually lower drug costs. Finally, former FBI Director and federal judge, Louis Freeh, released the findings of an investigation, with a more recent addendum, that note the ways drug importation would affect public health, safety and law enforcement.

Robust Research Funding

Life Sciences PA is a strong supporter of robust research funding that is the foundation for innovation in the life sciences. As home to two of the top-twelve recipients of National Institutes of Health (NIH) funding – the University of Pennsylvania and the University of Pittsburgh – Pennsylvania is one the U.S. leaders in basic research to advance human health. In 2021 Pennsylvania research institutions and small companies received more than $2 billion in NIH funding. This critical funding provides our ecosystem funding for research that private investors would find far too risky, and it is the launching point for the science that has led to a number of new therapies, cures, and technologies.

Additionally, Life Sciences PA supports the Small Business Innovation Research (SBIR) and Small Business Technology Transfer (STTR) programs, created in 1982 and 1992, respectively. While not life sciences specific, these two programs fund domestic small businesses engaging in research and development on the path to technology commercialization. In 2021, the SBIR program funded more than $30 million in life sciences research in Pennsylvania. This funding has helped many of our small member companies grow and thrive. Life Sciences PA supports a permanent reauthorization of this program in 2022.

Medical Devices

With more than 330 medical device and diagnostic companies, Pennsylvania is home to a significant number of entities working on developing new technologies to help patients live longer, healthier lives. As innovators and manufacturers, these companies benefit from policies that incentivize both of those endeavors.

To that end, and since the permanent repeal of the Medical Device Tax in 2019, Life Sciences PA has focused its efforts on support for a coverage pathway that would align reimbursement from the Center for Medicare and Medicaid Services with approval for a “breakthrough device.” The change will provide patients access to innovative medical technologies and ensure medical technology companies, many of which are pre-revenue, have a market through which to disburse their technology – a key component to generate continued investment in their research and development of innovate medical technologies. Unless reimbursement through Medicare or a commercial insurer is in place medical providers will not purchase and use the new medical device, which could prevent patient access to lifesaving technology. Without reimbursement, a “breakthrough” device and the company manufacturing it could face future

research and commercialization delays after having already spent many years and millions of dollars developing their medical technology.

Intellectual Property

Strong intellectual property protections are the foundation for innovation in the life sciences. The U.S. patent system, though now ranked 12th worldwide by the U.S. Chamber of Commerce, maintains important protections for companies researching and developing new medicines and technologies. These protections are especially important for the many small life sciences companies that call Pennsylvania home, and for the technology transfer offices that many of our academic institutions operate to move inventions from the lab to widely available treatments and cures for patients.

For these reasons, Life Sciences PA opposes measure such as compulsory licensing and march-in rights that undermine innovative companies and the substantial research and development resources needed to bring novel therapies to patients. Specifically, Life Sciences PA opposed the Administration's support for Trade-Related Aspects of Intellectual Property Rights (TRIPS) waiver filed with the World Trade Organization (WTO). Strong and predictable IP systems helped accelerate the translational research occurring at academic research institutions to the private sector and changes to those protections could upend U.S. innovation in the life sciences.

Orphan Drug Act and Tax Credit

A rare disease is defined as one that affects fewer than 200,000 people. The process of taking a rare disease medicine from research through development and approval for patients has many unique challenges. With small patient populations, the development of medicines for rare diseases is significantly more difficult, costly, and risky than typical drug research and development.

In response to these challenges Congress passed the *Orphan Drug Act* in 1983 to promote investment in the development of medical solutions for rare diseases. This act provides companies and researchers incentives such as seven years of exclusivity and a 25% tax credit for qualified clinical testing expenses. In 1983 only 38 drugs had been approved for rare diseases. Since the passage of the *Orphan Drug Act* more than 650 therapies have been approved for rare diseases.

Life Sciences PA supports the Orphan Drug Act and opposes changes to it, such as the halving of the tax credit from 50% to 25% in 2017 and other modifications that would divert investment away from rare disease research.