U.S. Senate Committee on Appropriations
Full Committee Hearing
"U.S. Government Response: Fighting Ebola & Protecting America"
Testimony: Biotechnology Industry Organization
Wednesday, November 12, 2014, 2:00 p.m.

Introduction

Chairwoman Mikulski, Ranking Member Shelby, Members of the Committee, thank you for the opportunity to provide written testimony on the importance of robust funding for government programs that partner with industry to strengthen our national preparedness during events such as the current Ebola outbreak.

The Biotechnology Industry Organization (BIO) represents more than 1,000 companies, academic institutions, state biotechnology centers and related organizations across the United States and in more than 30 other nations. In the area of biodefense and public health preparedness, BIO represents a broad mix of small, medium and large companies involved in the research, development and manufacture of medical countermeasures (i.e. drugs, vaccines, diagnostics and devices) that will save lives in the event of a naturally-occurring outbreak, like Ebola or pandemic influenza, or a deliberate chemical, biological, radiological or nuclear (CBRN) attack.

Importance of the Public/Private Partnership – BioShield & BARDA

As the number of Ebola cases in West Africa continues to climb, there has been increased public focus on the need for vaccines and therapies to prevent and treat this extremely infectious and deadly disease. Before this epidemic, Ebola was largely considered a bioterrorist threat rather than a public health threat, as previous outbreaks of the virus had been small and isolated. There have been more cases and deaths in this outbreak than all others combined since the disease first appeared in 1976.

Without a commercial market, the development and manufacture of many medical countermeasures (MCMs), like those against Ebola, require a public/private partnership between the government and industry. A decade ago, Congress recognized this and created Project BioShield to serve as a government marketplace for MCMs. The BioShield Special Reserve Fund (SRF), which was originally funded through a ten-year advance appropriation of $5.6 billion, successfully drove innovation in the field of biodefense by providing a stable source of funding which signaled to potential private sector partners that a reliable market was in place for MCMs. As a result, over 70 companies and institutions have partnered with the U.S. government and advanced their MCM research and development (R&D) programs, and the advanced development pipeline has supported over 85 MCM candidates. More than 50 million doses of vaccines and drugs against anthrax, botulinum toxin, smallpox, and radiological threats have been developed and procured through Project BioShield and now stand ready for deployment in the Centers for Disease Control and Prevention's (CDC's) Strategic National Stockpile (SNS).

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The Biomedical Advanced Research and Development Authority (BARDA) has also played a critical role in this public/private partnership. Congress established BARDA in 2006 to provide additional funding and support during late stage MCM development. The later stages of product development, often called the "valley of death," involve the greatest requirements for financial support and technical assistance, and the greatest technical failure. This support is particularly critical for products that may have both MCM indications and limited commercial indications, such as some antibiotics. The BARDA advanced development pipeline currently includes a wide range of MCM candidates, such as broad-spectrum antimicrobials, rapid diagnostics, and next-generation products.

Similar to Project BioShield, a government marketplace was created for pandemic influenza MCMs through multi-year, multi-billion dollar supplemental appropriations during the past decade. This funding has supported the advanced development of vaccines, antivirals, and diagnostics; pre-pandemic rapid response; and the replenishment of pre-pandemic stockpiles. As a result, the nation was able to mount an effective response to the H1N1 pandemic of 2009-2010.

Unfortunately, these guaranteed marketplaces ceased to exist at the end of fiscal year (FY) 2013 when BioShield funding expired and the supplemental pandemic influenza balances were exhausted. Both programs became subject to annual appropriations in FY 2014 and experienced a dramatic decrease in funding. The amount appropriated for Project BioShield, $255 million, does not reflect the authorization level passed by Congress in the 2013 Pandemic and All-Hazards Preparedness Reauthorization Act (PAHPRA), which is $2.8 billion over a five-year period. For pandemic influenza preparedness, only $115 million was appropriated by Congress. These funding levels create significant uncertainty for companies engaged in the research, development, and manufacture of MCMs, and in the case of Project BioShield, undermine the intent of the program.

MCM Development Is Complex & Risky

Biotechnology companies consider the public/private partnership and the resources available through BARDA, BioShield, and the pandemic influenza preparedness program when considering whether or not to pursue MCM development. The development of MCMs is a unique, resource-intensive, and complex process that can be costly and fraught with risk. Similar to commercial products, new countermeasures can take 8-12 years to develop at a cost of $800 million to $1 billion, and failure is common at all stages of development. Yet in most other ways MCM development and approval is much more complicated. Testing MCMs requires the use of multiple animal models to prove efficacy, which adds an extra dimension of risk and uncertainty to the process. If our collective goal is the use of innovative technology to solve these vital national security issues, then there must be acknowledgement of the higher degree of risk and uncertainty inherent in MCM development.

The time and company resources allocated for these products requires R&D and manufacturing resources that could otherwise be deployed on commercial products, and

therefore must be subjected to the same rate of return analysis. In addition, private investors place little to no value on this type of research as the market is difficult to calculate and the guarantee of government purchase is not always clear. Therefore, there are very limited external private funds to support companies in the MCM space, and the funding appropriated by Congress is critical to advance these products.

Robust Funding Is Critical for the MCM Enterprise

To encourage companies to maintain and/or expand their commitments to MCM development and manufacture, robust and stable funding for federal biodefense programs is needed. BIO strongly urges the Committee to fund BARDA and Project BioShield at the levels authorized in PAHPRA. For BARDA, this means continuing to appropriate $415 million annually, and for BioShield, appropriating the remainder of the $2.8 billion for use through FY 2018. BIO also supports an annual appropriation of $330 million for pandemic influenza MCMs, and a minimum of $543 million for the SNS in FY 2015 with significant increases in FY 2016 and subsequent fiscal years when procurement of additional MCMs will be shifted from Project BioShield to the stockpile.

At these levels, the government can truly take an all-hazards approach to health security and public health preparedness by funding the advanced development and procurement of MCMs for a range of threats simultaneously. Further, these funding levels allow BARDA the flexibility to quickly pivot and allocate resources as needed, such as in the case of an event such as the current Ebola epidemic. These funding commitments would also provide incentives and certainty to private companies that are investing or considering investing in the MCM space.

BIO supports the Administration's emergency funding request to enhance the government's response to Ebola at home and abroad. The $157 million in additional funding for BARDA is critical, particularly considering the significant expense of funding clinical trials for a number of candidate vaccines and therapies. Yet, we urge the Committee to consider enhancing regular appropriations for BARDA and BioShield for the remainder of FY 2015 to support not only the advanced development and procurement of Ebola products, but also for promising MCMs currently in pipeline to address other urgent threats.

Publication of a five-year budget plan for the entire MCM enterprise would further help demonstrate the government's commitment to the MCM enterprise. The Assistant Secretary of Preparedness and Response (ASPR) is required by law (PAHPRA) to develop this plan, which is meant to inform prioritization of resources, identify MCM life-cycle costs, and outline how BARDA proposes to spend the $2.8 billion for the SRF between FY 2014-2018, if fully appropriated. Because the budget plan has not been released, it is impossible for companies to gauge potential funding opportunities for FY 2015 and beyond and plan their business accordingly. In a report released in December 2013, the Government Accountability Office (GAO) called for the public release of the budget plan, stating that "providing estimates would allow HHS's industry partners to suitably target

research and development to fulfill countermeasure priorities, especially in tighter budget climates."

BIO respectfully requests that Congress urge both the ASPR and the Office of Management and Budget to expedite the release of the five-year budget plan for the entire MCM enterprise.

Conclusion

The public-private partnership so vital to the MCM enterprise has been a success to date. It has significantly enhanced preparedness for national security threats by developing products, delivering stockpiles, building infrastructure, and driving innovation. However, sustainability of the MCM market is absolutely critical if we as a nation are to maintain our current state of preparedness and continue to build upon it.

While the magnitude of the current Ebola epidemic in West Africa was unexpected, Ebola has been on the Department of Homeland Security's material threat list and considered a bioterrorist threat for years. The next naturally-occurring outbreak or deliberate attack may involve a threat that is completely unknown to us.

To prepare for the full range of potential threats, we must prioritize funding for Project BioShield, BARDA, pandemic influenza, the SNS, and other programs that are essential to public health preparedness this year and in coming years. These programs simply cannot be funded only after a disaster hits. BIO commends the Committee for holding this important hearing and recognizing the critical role of these programs in protecting Americans. BIO stands ready to work with Congress to strengthen our nation's security and public health preparedness.