

Hearing Testimony

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Preparedness, Response and Communications Subcommittee

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Good morning Chairman Bilirakis, Ranking Member Richardson, Members of the Committee, ladies and gentleman. I am Phyllis Arthur, Senior Director for Vaccines, Immunotherapeutics and Diagnostics Policy at the Biotechnology Industry Organization (BIO). BIO represents more than 1,100 companies, academic institutions, state biotechnology centers and related organizations in all 50 states.

In the area of biodefense, BIO represents a broad mix of small, medium and large companies involved in the research, development and manufacturing of medical countermeasures or MCM's. These companies develop and manufacture biological products for the detection, diagnosis, treatment, prevention and delivery of countermeasures in response to chemical, biological, radiological and nuclear events.

Ensuring the availability of MCM's that will save lives during a public health crisis (such as pandemic influenza) or weapons of mass destruction attack (such as anthrax) is the responsibility of the U.S. government. BIO and its members were therefore encouraged when Secretary Sebelius engaged the Department of Health and Human Services (HHS) in an intense review of the Public Health and Emergency Preparedness Enterprise (PHEMCE). BIO actively engaged in this process, participating in stakeholder meetings related to most facets of the Enterprise. Some of the recommendations from industry were incorporated into the final review and still others can be included in the upcoming reauthorization of the Pandemic and All-Hazards Preparedness Act (PAHPA) or other biodefense related vehicles moving through Congress.

The lack of a viable commercial market for most of these products necessitates the active engagement of the government in the development of these essential products. Over the last ten years, bipartisan Congressional efforts have created and funded an Enterprise that has begun to show success. In the past 2 years, several key countermeasures in the area of smallpox and anthrax have been delivered to the Strategic National Stockpile. Furthermore several key procurement contracts have been issued that will lead to the final development of other countermeasures. Future plans and investments are pivotal to

continue that success and further strengthen and improve the responsiveness of the United States.

One of the goals of the U.S. government in conducting the MCM review was to identify and solve those issues limiting companies of all sizes from successfully engaging in the countermeasures process. BIO identified three core issues that have limited industry's participation in PHEMCE. These issues fall into three categories: (1) defining a viable market value for MCMs versus the opportunity cost of investing in a different area; (2) management of cost and risk, especially in the regulatory process; and (3) sustainability of the market over time.

(1) Defining a Viable Market Value for MCMs

The Project BioShield Act of 2004 accomplished several important goals, but the most significant part was the creation of the Special Reserve Fund (SRF). BioShield is designed to guarantee companies that the government will purchase new, successfully developed countermeasures for placement in the Strategic National Stockpile (SNS). Annual appropriations to the Biomedical Advanced Research and Development Authority (BARDA), which was created in 2006 and manages Project Bioshield and PHEMCE, and the existence of the SRF, define the marketplace for MCM's. Companies consider the amount of resources available through BARDA and the SRF when comparing the opportunity cost of pursuing the development of a specific countermeasure. The time, and company funds, spent on these products diverts R&D and manufacturing resources away from commercial products and must be subjected to the same rates 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 uncertain. Therefore, there are very limited private sector funds to support companies in the MCM space.

Part of the opportunity cost assessed by industry is the time required to achieve success. While industry, particularly small biotechnology companies, finds BARDA an increasingly desirable and effective partner in advanced development, the acquisition and contracting functions to acquire new countermeasures are viewed as lengthy, opaque, and unpredictable. The trigger to transition a program from advanced development to procurement is unclear. Target dates to complete contract awards are typically not met, some acquisitions are delayed by years or canceled. The negotiation process is needlessly lengthy with technical and security issues resolved prior to pricing discussions. The rationale and potential triggers for contract options are unclear. Lastly, while Federal Acquisition Regulations (FAR § 12.102(f)(1)) states that contracting officers "may treat any acquisition of supplies or services that, as determined by the head of the agency, are to be used to facilitate defense against or recovery from nuclear, biological, chemical, or radiological attack, as an acquisition of commercial items," not a single novel countermeasure has been designated as a commercial item. The signal to industry is that despite the enormous risks of development of novel countermeasures, pricing of new drugs and vaccines developed as countermeasures, is far below that in commercial markets.

(2) Management of Cost and Risk and the Regulatory Process for MCMs

The development of countermeasures is a unique, resource-intensive and complex process that can be costly and fraught with risk. One of the most significant risks is that countermeasures are approved via a convoluted regulatory pathway. In many respects the regulatory process for MCM's is no different from commercial biologicals. Products 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 other ways MCM development and approval is much more complicated. The required use of animal models to prove efficacy adds an extra dimension of risk and uncertainty to this process.

The coordination and collaboration between the various government agencies involved in the Enterprise can add to the overall uncertainty surrounding MCM's. The prioritization of threats is not transparent so it is not clear which pathogens, platforms, indications and target populations are the most important. Indeed one government agency may view these threats in different ways from the others, thus leading to conflicting or overlapping programs with differing priorities. While BARDA and its Department of Defense counterparts have been working more collaboratively to coordinate their requirements, the FDA has not been as involved in the discussion of threats or in the early development of these requirements. The lack of full integration across the Enterprise, especially as it pertains to the approval process for countermeasures, has, in several instances, led to significant delays and new regulatory actions by companies in order to achieve licensure for a product.

One of the most significant recommendations from the PHEMCE review was the recommendation to invest significantly in the FDA review and regulatory science processes. This is a recommendation that is strongly supported by BIO and its members. The FDA has tremendous expertise in the science of drug development and the manufacturing of complex drugs, diagnostics and biologics. Effectively integrating FDA into the MCM development efforts will ensure that the government can have more rapid access to fully licensed medicines, devices and diagnostics for national security threats in a cost-effective manner.

To meet this goal FDA needs to be given an affirmative role in solving the scientific and regulatory hurdles, not just the review and approval, of MCM's. This can best be accomplished by encouraging the FDA to work collaboratively with company sponsors to design development plans and associated studies, especially those requiring use of animal models. The current structure and resources provide a disincentive for FDA to spend time on these complex issues in partnership with industry. Additionally, FDA funding targeted to improving MCM efforts should be linked to measurable metrics.

BIO recommends that the FDA become more involved in the development of MCM's through a combination of planning and coordination activities and implementation of specific measurements for MCM initiatives.

(3) Sustainability of the MCM Market

The Project BioShield Act and PAHPA helped to build processes to advance clinical and manufacturing infrastructure to protect against a multitude of biological threats. While there have been successes in several strategic portfolios within HHS, currently the U.S. is decades away from having an adequate arsenal of countermeasures to safeguard our citizens. In addition to developing and stockpiling countermeasures against currently anticipated threats, it is critical that the U.S. builds capability to respond to new threats such as newly emerging diseases and genetically-modified pathogens.

The reauthorization of PAHPA and the replenishment of the BioShield SRF are critical to these efforts. Therefore BIO strongly urges Congress to replenish the Special Reserve Fund simultaneously with the reauthorization of PAHPA. The SRF should be funded at a level that incentivizes private industry to actively participate in the MCM process.

The PHEMCE review highlighted the importance of a 5-year plan for the Enterprise with goals tied to measurable outputs and outcomes. Due to the long development timelines for biological products, industry partners need to be able to plan and communicate with their investors on the anticipated value and impact of its MCM projects with some increased level of certainty. BIO recommends that Congress formally establish a process by which HHS and its relevant agencies (NIH, CDC, FDA and ASPR) develop an integrated five-year plan that can be shared with all stakeholders. A systematic, transparent vision from the U.S. government will help companies assess the viability of both their existing and future countermeasures' programs. This \ multi-year strategic plan, coupled with modifications to the contracting processes, could encourage increased industry participation.

Lastly, one of the most critical parts of responsiveness involves the nation's ability to detect and identify these threats to best mount the proper and timely response. BIO members are also concerned that the U.S. government make the right investments in global and U.S. surveillance testing and reporting networks. Efforts should be made to extend the network and invest and explore common platforms and design tools that can increase efficiency and reduce costs. Improving interagency coordination within the U.S. national network, while striving to modernize its technical and technological capabilities, would increase speed and accuracy in detecting emerging diseases and threats.

BIO commends the Committee for holding this important hearing and stands ready to work with Congress on these important issues. Ensuring the availability of MCMs is a critical responsibility of the U.S. Government. The lack of viable commercial markets for these products necessitates the active engagement of government in supporting the development of these essential products. Over the last ten years, bipartisan Congressional efforts have created and funded a public health emergency medical

countermeasure enterprise (PHEMCE) that has begun to show success. Future plans and investments are essential to this effort.

Congress has the opportunity to implement changes to the PHEMCE that will improve preparedness, accelerate approvals and reduce the cost of developing essential medical countermeasures, including medical devices, and we look forward to working together with you in these efforts.