

# The State of Biosecurity in 2008 and Proposals for a Public/Private Pathway Forward

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## Background

The Alliance for Biosecurity, a collaboration between the Center for Biosecurity of the University of Pittsburgh Medical Center and the below listed biopharmaceutical companies, seeks to engage government and other domestic and international stakeholders in a creative dialogue aimed at improving and accelerating efforts to develop medical countermeasures (MCMs) for the nation's Strategic National Stockpile (SNS). This document assesses accomplishments to date, identifies existing challenges to the development and procurement of additional countermeasures, and briefly outlines proposals for overcoming the many challenges to achieving a successful and sustainable biodefense program in the US. **The Alliance looks forward to opportunities to explore these and other proposals with those in government devoted to leading national biosecurity initiatives.**

## Status of Biodefense Preparedness

The Project BioShield Act of 2004 authorized \$5.6 billion dollars over 10 years to purchase MCMs for the SNS. To date, there are BioShield contracts for the procurement of the currently-licensed anthrax vaccine, therapeutic antibodies for inhalational anthrax, a botulism heptavalent antitoxin, a smallpox vaccine, and several products for radiological and nuclear threats, obligating a total of about \$1.9 billion of the \$5.6 billion BioShield fund. Procurement of these particular products will go a long way to fulfilling the needs for vaccine and therapeutic countermeasures to address the highest priority threats for which products in advanced state of development, or licensed products, already exist. For the most part, the products under contract for procurement are based on existing technologies and may under-represent the breadth of products that will be needed for a comprehensive biodefense stockpile.

In 2006 the Pandemic and All-Hazards Preparedness Act (PAHPA) established within HHS the Biomedical Advanced Research and Development Authority (BARDA) to help coordinate federal research and development of MCMs and to provide much needed support for advanced research and development. BARDA was authorized at \$1.07 billion over three years, but only \$201 million has been provided by Congress through FY 2008, approximately one-fifth of the authorized level. The first round of advanced development contracts partially funded by BARDA and awarded through NIH – for countermeasures addressing anthrax, plague and tularemia – were issued in the Fall of 2007. The second round of BARDA advanced research and development contracts are expected in late 2008.

HHS's Public Health Emergency Medical Countermeasure Enterprise Implementation Plan for Chemical, Biological, Radiological, and Nuclear (CBRN) Threats (PHEMCE Plan) identified, in April of 2007, the proposed near-term and mid-term acquisition needs of the government to address CBRN threat agents. The PHEMCE Plan identifies high-priority CBRN threats and confirms the need to acquire eight new biodefense MCMs (i.e., five small molecule drugs, two vaccines, and one

## Alliance for Biosecurity

Bavarian-Nordic ▪ Cangene Corporation ▪ DOR BioPharma, Inc.  
Dynport Vaccine Company LLC, a CSC Company ▪ Center for Biosecurity of UPMC ▪ Elusys Therapeutics  
Emergent BioSolutions ▪ Hematech, Inc., a subsidiary of Kirin Pharma Company, Ltd. ▪ Human Genome Sciences, Inc. ▪ Intercell USA, Inc. ▪ NanoViricides, Inc. ▪ Pfizer Inc. ▪ PharmAthene ▪ Siga Technologies

biological therapeutic). This is in addition to diagnostics, biosimetry tests, CHEMPACKS, and countermeasures for radiological and nuclear threats. The PHEMCE Plan is intended to enhance the original BioShield program and assist MCM developers by further clarifying additional material threats and establish priorities for development of additional MCMs.

## **Cost of Preparedness**

The Center for Biosecurity has carried out an independent economic analysis that estimated the cost of BARDA's advanced development mission. The analysis found that it would require \$3.4 billion in FY 2009 to support one year of advanced development of a pipeline that would have a 90% chance of ultimately developing one successful MCM for each of the eight biodefense requirements in the PHEMCE Plan. This level of annual funding would need to be sustained for many years. If the \$102 million BARDA received in FY 2008 continues annually, that would only be sufficient for BARDA to support development of two countermeasure candidates each of which would only have a 30% chance of success. Clearly, there is not enough funding appropriated or authorized under BARDA to successfully support development of needed MCMs. Similarly, the original appropriation of \$5.6 billion for Project BioShield is equally insufficient to ensure that once MCMs are developed there will be funds available to procure them and maintain the stockpile.

## **Industry and Investor Engagement**

Interest of the public and private capital markets in biodefense has declined over the last 2-3 years. This decline has been attributed to a number of factors that include the growing skepticism over the commitment to sustainability of markets for drugs that are intended to be used only in the SNS. Project BioShield initially encouraged a number of small to medium-sized companies to invest in biodefense countermeasure development, but a number of those companies have abandoned their biodefense projects after losing corporate support or investor backing. Reservations about the government's long-term commitment to supporting biodefense countermeasure development have increased in response to insufficient federal appropriations for biodefense and general uncertainty about the size, nature, and sustainability of a government "market" for MCMs. Further, delays and modifications in procurement award timelines lend credibility to the view that the government market is unreliable from an investment point of view.

Companies that remain in the biodefense field, such as those in the Alliance, are intently following recent developments and looking for ways to partner with government to stimulate near-term successes. Such successes should include both BARDA advanced research and development contracts as well as procurement awards. Procurements are the only government action that allows companies to project sales and realize revenue — which are the metrics used by the investment community to evaluate companies. More predictable and frequent actions by government will inspire investors, Congress, and others to re-commit energy and funds to advance the country's biosecurity goals. An example of how BARDA could stimulate industry engagement would be for it to use its authority to engage in "other transactions" available to it under PAHPA. We encourage the BARDA office to explore all avenues open to it to increase the flexibility and speed of engagements with industry and to streamline and otherwise simplify the contracting and funding process for advanced development projects.

## **Top Priorities**

The Alliance for Biosecurity believes that the biodefense mission of the United States could be significantly bolstered and ultimately successful with a redoubled investment in leadership, coordination and communication, funding, and long-term vision. These top priority recommendations are outlined below.

## **1. Leadership**

The Alliance has long urged the government to establish and support a central point of leadership and accountability within HHS for biosecurity. Consequently, the Alliance is enthusiastic about the newly-appointed Director of the BARDA office. We urge the Director to use his authority to the full extent authorized by the PAHPA legislation to:

- Instill a sense of urgency for drug and vaccine development and procurement efforts both in government and in the private sector. Administration officials should be asked to support BARDA's mission by reinforcing this sense of urgency with Congress.
- Promote a risk-tolerant culture within the BARDA office that understands the realities, risks, timelines, and costs of drug development.
- Encourage BARDA and industry to support the investigation of innovative approaches for drug development and manufacturing.
- Focus more on the potential biothreats and the corresponding countermeasures, rather than the price tag. The likelihood of replenishment of the BioShield Special Reserve Fund and of higher BARDA Advanced Research and Development Fund appropriations will increase as successes are achieved.
- Increase the number of BARDA staff who have drug development and manufacturing experience.
- Take steps to streamline, improve, and accelerate the current RFP and contracting processes informed by strong two-way communication between the government and the private-sector to ensure realistic priorities and expectations for MCM development, based on the existing capabilities and technologies within industry, government and academia.
- Create a consistent funding cycle, similar to NIH and DoD (RFPs are issued and awarded with regularity), with flexibility for high priority items that is not tied to the annual appropriations cycle.
- Continue to develop long term, comprehensive biosecurity strategy and update the PHEMCE Plan with input from various stakeholders.
- Develop a culture that is focused on partnering with industry and academia.

## **2. Coordination and Communication**

The Alliance recommends that the BARDA office make clear to countermeasure developers the end to end strategy for development, procurement, and maintenance of each needed product and create a fully integrated and transparent process across government entities according to those strategies. The Alliance appreciates that a fruitful and continuous dialogue between government agencies and developers early in the process will provide the basis for increasing the chances of successful implementation of the prioritized countermeasures.

- For each identified countermeasure, there should be a tailored strategy that connects NIAID biodefense basic research funding with BARDA advanced development contracts and BioShield procurement contracts. While there can be no guarantee of procurement based on a NIAID grant or advanced development contract, such funding should have a direct connection to the intended procurement needs of government.
- The BARDA office should also take steps to coordinate with FDA on plans and processes for countermeasure evaluation and application. For instance, the

differences between HHS's procurement criteria using Emergency Use Authorization (EUA) and FDA's criteria for the issuance of EUAs should be clarified, resolved, and communicated.

- The BARDA office should continue to provide (through Stakeholder workshops and other commonly occurring public and private meetings) early guidance to industry about any emerging or shifting priorities, as many resources must be simultaneously mobilized to begin and to sustain countermeasures development.

The Alliance believes that each company pursuing a countermeasure product correspondingly has a responsibility to communicate proactively with its government partners. Industry must coordinate early and often with government, including the BARDA office, openly discuss technical, regulatory, and business challenges to development, and partner appropriately with relevant agencies. Biodefense is essentially a brand new industry, and companies involved have a responsibility to become well acquainted and compliant with the various laws and regulations applicable to biodefense government contracting. Companies must educate themselves about government's needs and seek to meet those requirements in an efficient way. It is incumbent on both industry and government partners to clearly articulate and discuss the key requirements early, starting at the time a formal requirement delineating the size of the threat, types of products needed, and the funding thresholds are established. Alliance members appreciate that the nation's biosecurity needs will only be met if they intimately understand those needs and are prepared to candidly discuss their technologies and capabilities to develop identified products and platform technologies.

### **3. Funding**

The enormous gulf between the procurement goals of the government and the level of government funding significantly handicaps the national biodefense MCM program. The Alliance, HHS, and the Administration all have an important role to play to lessen this gap.

- A mission of the Alliance for Biosecurity is educating members of Congress about (1) the national security threats posed by bioterrorism and naturally occurring pandemics; (2) the costs, risks, and timelines associated with drug development; and (3) the financial support needed by BARDA to achieve success.
- BARDA can demonstrate progress and encourage higher levels of appropriations in the future by putting its available funds to use now.
- The Administration could have the most influence over BARDA and BioShield funding levels by communicating the seriousness and reality of the threat and by making the case for adequate funding levels in Congress.

### **4. Long-Term Vision**

Now is the time for government and industry stakeholders to engage in a dialogue to address critical strategic issues that have not yet been resolved. It is a critical time for both government and industry, and it is critical that BARDA not lose momentum. There must be an effective transition strategy to ensure that the new administration understands the importance of the biodefense program and that new staff are educated quickly. Longer term two of the most pressing questions are (1) what are the government's plans for long term sustainability of the MCMs in the SNS and (2) how will the United States protect against new or variant infectious agents that could occur naturally or could be created through traditional microbiological or new genetic engineering techniques?

As for the first issue, the United States will need long-term access to manufacturing capability to produce more product when needed to replace expired MCMs and if more is urgently needed in the event of an attack. This is a resource-intensive but necessary component of national biosecurity efforts. It is essential to explore and assess available options and begin to develop a strategic approach for warm-base manufacturing and other approaches to guarantee the effectiveness of the SNS for years to come.

For the second issue, the Homeland Security Presidential Directive/HSPD-18 (Jan. 31, 2007), the PHEMCE Plan, and numerous biodefense experts acknowledge that the biothreats facing the country are not limited to those identified in the PHEMCE Plan. While BARDA is given direction within PAHPA to promote innovation (e.g., BARDA could fund development of broad-spectrum antibiotics and platform technologies), there is no current government or industry strategy to achieve this type of "flexible defense." A concerted, well-thought out approach that (i) communicates to industry that flexible defense is a priority for government, (ii) supports unique partnerships and collaborations, and (iii) provides adequate resources for promising endeavors would add more energy and reality to the important pursuit of integrative science, methods, and new technologies that will produce dynamic diagnostics, therapeutics, and prophylactics for future threat agents.

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