Testimony
Committee on Homeland Security
Subcommittee on Emerging Threats, Cybersecurity, and Science & Technology
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HHS Update on the Advanced Development and Procurement of Medical Countermeasures

Statement of
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Mr. Chairman and Members of the Subcommittee, I am Gerald Parker, Principal Deputy Assistant Secretary to the Assistant Secretary for Preparedness and Response. I am honored to be here today to speak with you about the accomplishments, challenges and plans for moving forward at the Department of Health and Human Services (HHS) with regard to our efforts to develop and acquire medical countermeasures for chemical, biological, radiological and nuclear (CBRN) threats, including the use of new authorities available under the Pandemic and All-Hazards Preparedness Act.

Today I will review the Department's Public Health Emergency Medical Countermeasures Enterprise (PHEMCE) Strategy and Implementation Plan, a comprehensive, intra-agency framework to guide the implementation of Project BioShield. Specifically, I will discuss the roadmap for the future of Project BioShield; the status of existing Project BioShield acquisitions; those lessons learned from previous acquisitions; and the path forward to enhancing our preparedness.

First, I would like to review briefly the recent progress the Department has made:

- The Secretary has convened a Department-wide team to execute an aggressive implementation plan for the Pandemic and All Hazards Preparedness Act (P.L. 109-417]. We have already made progress. As directed in the Act, the Department has successfully transferred the National Disaster Medical System. Also, Health Resources and Services Administration transfers are going smoothly. The BARDA organizational structure is set, with the BARDA Director reporting to the HHS Assistant Secretary of Preparedness and Response. We

hope to fill this position by the summer. We are planning to use advance and milestone payments. As you know, these new tools are critical for the manufacturing, approval, and delivery of a product into the Strategic National Stockpile (SNS) and to address advanced development – the middle phase of product development between applied research and acquisition commonly referred to as the "Valley of Death."

- In July of last year, the Department established the Public Health Emergency Medical Countermeasures Enterprise (PHEMCE), an intra-agency structure to coordinate all levels of public health preparedness efforts against terrorist and naturally occurring threats. The Department also released the PHEMCE Strategy in March and just released the PHEMCE Implementation Plan, which I will describe later in my testimony.

Public Health Emergency Medical Countermeasures Enterprise

On March 20th HHS released the Public Health Emergency Medical Countermeasures Enterprise (PHEMCE) Strategy, for which HHS solicited public comment and incorporated substantial stakeholder input. As you know, the Department has also just released the PHEMCE Implementation Plan, which identifies top priorities for medical countermeasure research, development and acquisition programs that HHS has determined, in collaboration with interagency partners, to have the greatest potential to improve public health emergency preparedness. Both provide a comprehensive framework that establishes the principles that HHS will employ, as well as specific

procurement priorities, to ensure the most appropriate medical countermeasures are developed and acquired to protect the American public.

One of the central goals of the PHEMC Enterprise is to enhance the transparency of the medical countermeasure development and acquisition process for researchers, industry stakeholders, state and local public health officials, and the public. The PHEMC Enterprise oversees every aspect of the preparedness process, from establishing requirements for medical countermeasures and supporting research and development, to acquiring, storing, maintaining and deploying these countermeasures to protect the American public in the event of a public health emergency.

Just recently, the Department released the PHEMCE Implementation Plan, a road map for the prioritization of future Project BioShield acquisitions of vaccines, therapeutics and diagnostics for chemical, biological, radiological, and nuclear (CBRN) threats. The implementation plan clarifies for industry and the general public the Department's priorities and expectations for medical countermeasure development and acquisition. The Department's PHEMCE Implementation Plan enhances transparency for BioShield stakeholders by identifying criteria for medical countermeasure candidates and detailing priorities for future medical countermeasure acquisitions. The Plan incorporates valuable lessons learned from the initial implementation of BioShield.

Status of BioShield Acquisitions

HHS has launched acquisition programs to address each of the Material Threat Determinations initially deemed by DHS to be threats to the U.S. population sufficient to

affect national security: radiological and nuclear agents; *Bacillus anthracis* (anthrax); smallpox virus; and botulinum toxin. These Project BioShield procurement activities are also highlighted in the Project BioShield 2004-2006 Annual Report to Congress. We continue to press forward to acquire all the medical countermeasures needed for our preparedness.

Radiological/Nuclear Agents

HHS is committed to developing and purchasing products to respond to radiological and nuclear threats. This includes products to address the impact on blood cells of Acute Radiation Syndrome (ARS). HHS will continue to pursue an initial acquisition of 100,000 treatment courses of a safe and effective medical countermeasure to treat the bone marrow suppression associated with ARS that would predispose affected individuals to severe infections and increased mortality.

As you know, HHS recently withdrew a request for proposals (RFP) for an Acute Radiation Syndrome medical countermeasure. The decision was made because no offeror responding to the RFP possessed a product that was sufficiently mature to qualify for a BioShield acquisition. We plan to move forward with a new RFP as quickly as possible. This RFP will take advantage of new authorities the Department has acquired under the Pandemic and All-Hazards Preparedness Act, as well as scientific advances and evolution of the marketplace since the previous RFP closed. In addition, HHS will support research and development using new authorities under the Pandemic and All-Hazards Preparedness Act, and will continue to support development of an ARS

countermeasure as well as other promising radiological/nuclear medical countermeasure candidates.

It is important to note that HHS has already made progress and has products in the SNS to deal with a radiological/nuclear threat today. For example, the SNS current inventory of medical countermeasures includes the chelating agent Prussian blue, which enhances the excretion of cesium-137, a radionuclide that could potentially be used as part of a dirty bomb. The SNS also contains enough potassium iodide or 'KI' tablets for use in the event of a release of radioiodines. KI can be used to reduce the risk of thyroid cancer in individuals or populations at risk for inhalation or ingestion of radioiodines, which may be one of the radionuclides released after an attack on or an accident at a nuclear power facility. Because children would be at special risk of thyroid cancer and other complications, in March 2005 the Department awarded a BioShield contract to Fleming & Company, Pharmaceuticals of Fenton, Missouri for the manufacture and delivery of 1.7 million doses of liquid potassium iodide (KI), a formulation that is more suitable for young children. In February 2006, the original contract was modified to acquire 3.1 million additional bottles of pediatric KI. Delivery of the first procurement is complete.

In early 2006, the Department awarded a BioShield contract to Akorn, Inc. of Buffalo Grove, Illinois for the manufacture and delivery of two approved medical countermeasures for radiological or nuclear incidents. Delivery to the SNS of over 475,000 doses of Calcium-DTPA (Diethylenetriamine pentaacetate) and Zinc-DTPA was completed in April 2006. These drugs will be used to treat victims internally

contaminated with transuranic radionuclides such as plutonium or americium. Such internal contamination could occur following the detonation of a dirty bomb or improvised nuclear device, the use of other types of radiological dispersion device, or a terrorist attack against stored radioactive material.

Finally, the SNS already holds thousands of courses of growth factors that could be useful for addressing the hematopoietic effects of ARS, as well as general supplies (such as antibiotics, anti-nausea drugs, and materials to treat burn/blast injuries) that would be required to treat the complex array of medical problems encountered following the detonation of an improvised nuclear weapon.

Bacillus anthracis

HHS works to ensure the nation maintains a comprehensive stockpile of vaccines, antibiotics, and antitoxins to be prepared against a future anthrax attack. Today, in the SNS, there are antibiotic tablets sufficient to provide 41 million regimens of prophylaxis and over tens of thousands of intravenous treatment regimens for bacterial threat agents including anthrax, plague and tularemia. Under Project BioShield contracts, two companies, Cangene and Human Genome Sciences, will provide additional therapeutic anthrax antitoxins to treat symptomatic patients. Delivery of the first of these new products is anticipated to begin this year.

In December 2006, the Department announced the termination of a contract with VaxGen for the delivery of 75 million doses of a second generation anthrax vaccine for failure to meet contract milestones. Anthrax vaccines are a key component of our

multifaceted preparedness program and we have delivered to the SNS 10 million doses of the currently available anthrax vaccine adsorbed (AVA).

HHS remains committed to the development and acquisition of a second generation anthrax vaccine, however, and has developed a comprehensive strategy for advanced development and acquisition of current and next generation anthrax vaccines that will continue to support the development of a second generation anthrax vaccine that is scientifically well-defined and well characterized. These interim steps will aid our progress in the development and acquisition of a third generation anthrax vaccine – preferably one that can be stored at room temperature and be self-administered, two product characteristics which are highly desirable during a public health emergency.

Smallpox virus

HHS has made significant progress in procuring smallpox vaccine for the SNS. The smallpox vaccine stockpile has grown from 90,000 ready-to-use doses in 2001 to enough vaccine to protect 300 million people, or every man, woman, and child in America. In addition, HHS expects to announce in the near future, the results of its RFP for a Modified Vaccinia Ankara (MVA) smallpox vaccine. The MVA vaccine may provide smallpox immunity for those with contraindications for receiving a live, replicating vaccine.

Botulinum toxins

In June 2006, HHS awarded a contract under Project BioShield to the Cangene Corporation for 200,000 doses of a despeciated equine heptavalent botulinum antitoxin,

which addresses all 7 types of botulinum toxin. The $363 million contract will expand greatly our existing stockpiles in the SNS.

Pandemic Influenza

In addition to Project BioShield acquisitions, the Department of Health and Human Services and its industry partners have made tremendous progress in developing and acquiring medical countermeasures to improve our preparedness for an influenza pandemic.

HHS is managing a robust and comprehensive medical countermeasures portfolio with over two dozen contracts – including an advanced research and development program as well as vaccine and antiviral stockpiles – and is initiating programs for building vaccine infrastructure.

Although much has been accomplished, continued vigilance and preparation are needed for us to be ready for Influenza – seasonal epidemics and Pandemics.

Lessons Learned and Path Forward

HHS has incorporated valuable lessons from the first two and a half years of BioShield and has applied these perspectives to the PHEMCE Strategy and Implementation Plan.

Funding

The fiscal year (FY) 2008 request for advanced development will help to bridge the gap between NIH research and development programs and Project BioShield, and it is critical to BARDA implementation.

It is helpful to review briefly the development and acquisition of public health medical countermeasures, which involve three broad steps. First, in the research phase, early studies are conducted to discover how disease occurs, and to identify candidate products to prevent or treat it. Second, during the advanced development stage candidate products must successfully navigate animal studies, several stages of clinical studies for safety and efficacy, and manufacturing scale-up leading to approval and licensure of a product. Third is acquisition, the stage in which a product is purchased by the federal government through Project BioShield.

Traditionally, basic research activities have been supported by research grants, primarily through the NIH. Acquisition is supported by the Special Reserve Fund (SRF) under Project BioShield and traditional SNS procurement mechanisms.

It is important to understand that prior to the enactment of the Pandemic and All-Hazards Preparedness Act, the SRF payment was conditioned upon delivery of a product to the stockpile. There was no defined mechanism to support advanced development. For small biotechnology companies, this stage of development, an inherently risky endeavor, usually relies on funding from venture capital or stock

offerings. Unfortunately, for biodefense medical products this stage has often proved challenging.

We are pleased that Congress has recognized the importance of advanced development in the establishment of BARDA. The President's FY 2008 Budget Request includes $189 million for advanced development that is critical to ensuring that Project BioShield is effective in making available appropriate medical countermeasures to protect against the most serious threats. Advanced development funding could help move promising MCM candidate products from research through the rigorous advanced development pipeline, to become eligible for procurement under the Project BioShield SRF.

In addition, HHS now has the authority to provide performance-based milestone payments in BioShield acquisition contracts.

BioShield Implementation

HHS recognizes that BioShield procurements must be made more swiftly. To help achieve this, HHS has established an interagency agreement with the Department of Homeland Security (DHS) to expedite the implementation of BioShield by clarifying roles and responsibilities and by establishing mechanisms to improve efficiencies. Furthermore, HHS is committed to shortening the time between the release of an RFP and the award of a contract.

Additional Lessons Learned

In the Department's Report to Congress on the Potential Barriers to Procurement, the Department identified potential barriers in four areas. First, the Department reviewed potential liabilities to industry, which was a major source of concern to companies and a recurring theme in the Project BioShield acquisition process. Second, the report discussed the opportunity costs of Project BioShield participation. As our experience to date demonstrates, acquisitions have not drawn the attention of large pharmaceutical or biotechnology firms. Third, risks inherent in product development are assessed, such as the award of contracts under Project BioShield before Food and Drug Administration (FDA) licensure, approval, and clearance. Finally, tradeoffs between timing and risk are identified, specifically the unique acquisition requirements for a Project BioShield to qualify for approval or licensure, given the limited odds of success for any product in the inherently risky development pipeline. According to an FDA study entitled *Challenge and Opportunity on the Critical Path to New Medical Products*, even for those products entering Phase 3 clinical trials, only 50 percent reach the market.

Transparency and Outreach

The Department is doing more to make the BioShield process transparent to stakeholders. The PHEMCE Strategy and Implementation Plan are the result of significant input from industry and other BioShield stakeholders for greater visibility into HHS requirements and priorities.

Project BioShield is a unique industry partnership. The Department is committed to fulfilling its roles both as a steward of the public's trust and as a reliable partner with

industry. As a good business partner, HHS is also working to enhance transparency to align industry expectations with the BioShield statutory authorities provided to the Department.

Our outreach with industry is critical to providing the visibility into Project BioShield that is necessary to ensure a mutual understanding between HHS and industry stakeholders, and to maximize participation. The Department continues to redouble its efforts at outreach with industry in a number of ways.

The annual Stakeholder Workshop informs stakeholders on the requirements, priorities and the most effective acquisition strategies. HHS will hold its second annual Stakeholder Workshop July 31 through August 2, 2007. This next Workshop will encompass BARDA, Project BioShield, and Pandemic Influenza, engage industry on the Department's present and future requirements, and solicit industry feedback. The BioShield Stakeholders Workshop held September of 2006 was hailed as a success by government and industry participants alike, and represents our intentions to maintain transparency and dialogue with our many partners in this effort.

As directed by the Pandemic and All-Hazards Preparedness Act, HHS is creating the National Biodefense Science Board (NBSB) to provide outside expert advice and guidance on scientific, technical and other matters of special interest to the Department regarding current and future CBRN agents, whether naturally occurring, accidental or deliberate. The Board will be employed as a mechanism to engage stakeholders, to provide a forum for the discussion and collaboration on controversial issues, and to

enhance transparency and credibility to the decision making process. Moreover, consistent with the Pandemic and All-Hazards Preparedness Act, the NBSB will include broad membership, including from industry, academia, the healthcare professional and consumer advocacy communities.

HHS is continually refining these processes both to ensure that stakeholders receive accurate, consistent, and timely information and to facilitate the participation of the largest number of biotechnology and pharmaceutical manufacturers.

Conclusion

The Department will use BARDA and BioShield acquisition authorities for advanced development, advance payments and performance-based milestones in future contracts. These authorities are critical to supporting an effective medical countermeasures enterprise.

This concludes my testimony. I will be happy to answer any questions.