Testimony

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HHS Progress in National Preparedness Efforts

Statement of

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Chairman Lieberman, Ranking Member Collins, and distinguished Members of the Committee. I am honored to be here today to discuss the progress of Department of Health and Human Services (HHS) to enhance public health preparedness and to review our vision for moving forward.

Today I will focus on three themes related to how HHS has made progress in our bioterrorism preparedness: (1) we have made significant acquisitions for the stockpile against the most serious threats facing the nation; (2) as a result of the lessons learned from previous acquisitions, we have changed the way we do business at HHS; and (3) we have taken an all-hazards approach to public health preparedness – the gains we make against each threat will help us across the spectrum of public health emergencies and disasters. I will also discuss our progress in implementing the Pandemic and All-Hazards Preparedness Act (PAHPA), detail lessons learned from our acquisitions to date, and discuss some of our upcoming activities.

However, I would like to be clear that medical countermeasure development and acquisition is only one component of our overall preparedness efforts, that fits into an all-hazards preparedness approach. Our all- hazards preparedness involves a shared responsibility among our entire Department, our partners in the International community, the Federal interagency, state, local, tribal and territorial governments, the private sector, and, ultimately, individuals and families. We are supporting state and local authorities through the State and Local and Hospital Preparedness programs to establish stockpiles of critical medical equipment and supplies, as well as for developing plans for response, maintenance and distribution countermeasures, and sharing of resources. The Department has effectively accomplished the transfer of the National Disaster Medical System (NDMS), and has aligned activities in the department to more effectively coordinate the preparedness and response enterprise, which focuses on the continuum of preparedness from research and development of medical countermeasures to response delivery platforms that support State and local authorities in dealing with the medical impacts of major disasters.

In addition, we have hosted multiple Department-wide exercises with senior leadership to test how we will leverage the full scope of HHS resources and capabilities in response to threats to public health, in addition to encouraging and engaging in State sponsored exercises taking place in their regions.

Medical Countermeasure Acquisition and Oversight

Our progress in securing medical countermeasures for the Strategic National Stockpile begins with and depends on effective planning. The central framework for medical countermeasures initiatives in the Federal government is the HHS Public Health Emergency Medical Countermeasures Enterprise (PHEMCE), established in July 2006. This coordinated interagency group is led by the Assistant Secretary for Preparedness and Response (ASPR), and includes the Centers for Disease Control and Prevention (CDC), the Food and Drug Administration (FDA), and the National Institutes of Health (NIH), and partners from the Department of Defense, Department of Homeland Security, and Department of Veteran Affairs. Through this Enterprise-wide effort, we are able to ensure that Federal activities with respect to needed medical countermeasures are effectively coordinated from research and development, to acquisition and ultimately deployment. This effort supports a range of programs that I will quickly summarize for acquiring medical countermeasures for manmade and naturally occurring public health threats.

Anthrax

Anthrax remains a top priority for the ongoing public health emergency preparedness efforts at HHS, and the Department is committed to developing and acquiring a robust, comprehensive portfolio of medical countermeasures against this threat.

Antibiotics represent the first line of defense to protect the nation following an anthrax attack. Today, we have over 40 million courses of antibiotics in the Strategic National Stockpile (SNS). Anthrax vaccines are also an essential element of our national

preparedness. It is possible that vaccines given as post-exposure prophylaxis in combination with antibiotics could provide longer-term protection, or allow for a reduction in the duration of the antibiotic regimen. HHS has awarded contracts for the acquisition of nearly 30 million doses of anthrax vaccine since 2005, including the recent contract award of 18.75 million doses of Anthrax Vaccine Adsorbed (AVA, BioThrax™). In addition, antitoxins are necessary to treat individuals with advanced stages of infection, and may contribute to a more successful therapeutic outcome. HHS has awarded contracts to two manufacturers to deliver antitoxins sufficient for treating 30,000 people. These vaccine and antitoxin contracts were awarded under the authorities of the Project BioShield Act of 2004. In addition, three BARDA contracts for the advanced development of other anthrax therapeutic candidates were recently awarded through a partnership with the National Institute of Allergies and Infectious Diseases (NIAID).

HHS remains committed to the development and acquisition of a second generation anthrax vaccine. While procuring and continuing to improve the currently available anthrax vaccine, HHS is using its new BARDA authorities to invest over $40 million in the continued development of an rPA anthrax vaccine. This investment builds on the rPA vaccine program that has been ongoing at the National Institute of Allergy and Infectious Diseases (NIAID) since 2002. BARDA is also finalizing a Request for Proposals (RFP) for an upcoming rPA vaccine acquisition. In addition, BARDA and NIAID released a Broad Agency Announcement in September 2007 for vaccine enhancement that will support important improvements in storage conditions and administration for vaccines against a wide array of biological threats.

Smallpox virus

In June 2007, BARDA awarded a contract for a next generation modified vaccinia Ankara (MVA) smallpox vaccine using performance-based milestone payments. The SNS currently contains sufficient smallpox vaccine for every American. HHS has also procured ACAM-2000, a live, single-dose smallpox vaccine developed by Acambis in

collaboration with CDC. This represents the first new biodefense vaccine to be approved by the FDA.

Botulinum toxin

In June 2006, HHS awarded a contract under Project BioShield to the Cangene Corporation for 200,000 doses of a botulinum antitoxin that targets all 7 serotypes of botulinum toxin. The $363 million contract will expand greatly our existing stockpiles in the SNS.

Radiological/Nuclear

We hold significant stockpiles of supplies to treat many of the complex array of medical problems following a potential radiological or nuclear attack, including antibiotics, anti-nausea drugs, and large quantities of supplies to treat burn and blast injuries. We have procured medical countermeasures to mitigate the effects of radiation exposure from either dirty bomb scenarios (Prussian blue and DTPA) or resulting from accidents or deliberate attacks involving nuclear power plants (potassium iodide (KI) in both pill and liquid form). HHS continues to pursue development and an initial acquisition of therapeutics to treat the effects of bone marrow suppression associated with the acute radiation syndrome (ARS) that might result from a nuclear blast. BARDA is also partnering with NIAID to fund advanced development of these medical countermeasures and for necessary testing facilities.

Pandemic Influenza

Since the emergency pandemic supplemental in December 2005, the scientists and public health experts at HHS have built an aggressive and broad-based medical countermeasures program for pandemic influenza. Congress has supported these efforts by allocating over $5.6B to the pandemic influenza preparedness efforts. This has allowed for a robust end-to-end approach that supports acquisition of existing products, research and development projects to produce next-generation countermeasures, and the retrofitting and construction of the facilities necessary to produce pandemic influenza vaccines. In particular, the pandemic influenza program is

focused on vaccines, antivirals, diagnostics, and non-pharmaceutical countermeasures. The President's FY08 Budget includes nearly $1.2 billion to further improve the Nation's preparedness, including $870 million for the development of vaccines and rapid diagnostics and the acquisition of additional antivirals.

With respect to vaccines, HHS has a number of efforts underway. These efforts supported the first U.S. licensure of an H5N1 vaccine in April 2007. By the end of 2007, HHS will have procured 26 million doses of pre-pandemic H5N1 vaccines. However, maintaining a domestic production capability for these priority countermeasures is also an essential component of the pandemic influenza preparedness strategy. Accordingly, in May 2007, we awarded two contracts for the retrofitting of existing domestic biological manufacturing facilities to produce egg-based influenza vaccines, and included warm base operations for up to five years. Finally, a strong advanced development program has led to the rapid maturation of next-generation vaccine production technologies, potentially supporting a shift from egg-based vaccine manufacturing to more flexible cell-based platforms. We anticipate a contract solicitation in 2008 to establish new domestic cell-based influenza vaccine manufacturing facilities that could produce at least 150 million doses of pandemic vaccine within six months.

Antivirals have become an increasingly important medical countermeasure for influenza. Today, the SNS contains 37.5 million treatment courses of antiviral drugs, and we will achieve our goal of 50 million treatment courses in 2008. HHS has also supported antiviral stockpiling at the state level. Through a federally subsidized program, states have purchased 15.1 million treatment courses of influenza antivirals to date and are expected to reach our goal of 31 million courses by July 2008.

The nature of severe influenza infections has also required us to focus on preparedness through non-pharmaceutical countermeasures, such as the essential role that ventilators play in the health care of critically ill patients. We are purchasing 2000 new

ventilators in 2007 for distribution during a pandemic, and there are opportunities for states to also invest in ventilator procurements. Developing ventilators that are more amenable to public health emergency use is a priority for advanced development. This presents a prime example of the integrative, all-hazards approach that the PHEMC Enterprise seeks. A more portable and easier to use ventilator could be an essential tool for responding to many different public health threats, when having a sufficient supply of ventilators could have an impact on the morbidity and mortality of exposure.

Pandemic and All-Hazards Preparedness Act Implementation

The Department has made significant progress in the implementation of PAHPA, which has resulted in tangible successes in the development and purchase of the necessary vaccines, drugs, therapies and diagnostic tools for public health emergencies.

Biomedical Advanced Research Authority

HHS has established the Biomedical Advanced Research and Development Authority (BARDA) to direct and coordinate the Department's countermeasure and product advanced research and development activities. In support of the mission and priorities of the HHS Public Health Emergency Medical Countermeasure Enterprise, (PHEMCE), BARDA establishes systems that encourage and facilitate the development and acquisition of medical countermeasures such as vaccines, therapeutics, and diagnostics, as well as innovative approaches to meet the threat of chemical, biological, radiological and nuclear (CBRN) agents and emerging infectious diseases, including pandemic influenza. BARDA provides an integrated, systematic approach to the development and purchase of the necessary vaccines, drugs, therapies and diagnostic tools for public health emergencies. It directs and coordinates the Department's countermeasure and product advanced research and development activities, including strategic planning for medical countermeasure research, development, and procurement.

Advanced Development

The Department has focused efforts on advanced development (AD) of promising medical countermeasure candidates for licensure. Advanced development support is necessary to move promising MCM candidate products from research through the rigorous advanced development pipeline to become eligible for procurement under the Project BioShield Special Reserve Fund (SRF). This activity is critical to bridge the gap from basic research and early development to procurement, to support multiple candidate products against multiple threats, to mitigate risk for Project BioShield acquisitions, and to successfully achieve the goals of BARDA.

BARDA and NIAID established a Memorandum of Understanding in FY2007 to jointly fund and manage the development of candidate medical countermeasures for CBRN agents. The first use of AD funds employed $99 million that was appropriated on May 25, 2007. In September 2007, the BARDA-NIAID partnership awarded contracts for the advanced development of anthrax antitoxins, anthrax rPA vaccine, smallpox antivirals, novel antibiotic formulations, and oral formulations of DTPA, a radiological/nuclear medical countermeasure.

Milestone Payment Authorities

Authorities for the award milestone payments in fulfillment of drug development goals provides two benefits: it allows the government to share drug development risks with manufacturers; and to closely monitor a company's progress from an earlier stage in drug development. PAHPA amended Section 319F-2 of the Public Health Service Act to allow milestone-based awards and payments for up to 50 percent of the total amount of a Project BioShield contract. In June 2007, BARDA awarded a contract employing these authorities for performance-based milestone payments for a next generation modified vaccinia Ankara (MVA) smallpox vaccine.

Stakeholder Outreach

HHS hosts regular meetings with representatives from relevant industries, academia, other Federal agencies, and international agencies. The 2007 PHEMCE Stakeholders Workshop, an annual event, was held July 31 through August 2, 2007. This Workshop encompassed BARDA, Project BioShield, and Pandemic Influenza, engaged with industry on the Department's present and future requirements, and solicited stakeholder feedback. The Workshop represents our intentions to maintain transparency and dialogue with our many partners in this effort. The first BARDA Industry Day was held on August 3, 2007, and provided an opportunity for stakeholders to demonstrate the operation and effectiveness of relevant countermeasure technologies that will be repeated in conjunction with future Stakeholder Workshops. ASPR will continue to engage with stakeholders regarding the implementation of the PAHPA legislation, and our next stakeholder meeting is scheduled for November 8.

National Biodefense Science Board

On May 24, 2007, the Secretary established the National Biodefense Science Board (NBSB) to provide expert advice and guidance to the Secretary on scientific, technical and other matters of special interest to HHS regarding activities to prevent, prepare for and respond to adverse health effects of public health emergencies resulting from current and future CBRN agents, whether naturally occurring, accidental, or deliberate. The NBSB will convene twice a year, and 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.

Lessons Learned and Path Forward

HHS has incorporated valuable lessons from the three years of BioShield and has applied these perspectives to the current and future medical countermeasure

development and acquisition projects. While we have achieved successes, we have also learned valuable lessons. The discovery and development of new medical countermeasures is complex and an inherently risky endeavor. The termination of the contract to procure an rPA anthrax vaccine exemplifies the multi-factorial challenges encountered in the implementation of Project BioShield. There are three particular lessons to be gained from recent Project BioShield procurements:

- First, for the most part, experienced and well-resourced companies have not responded to BioShield, and contract terms dictated by the BioShield statute were challenging, particularly for less experienced companies.
- Second, it is critical that developers establish effective relationships with the FDA early, to gain a clear understanding of the regulatory requirements with respect to their product for the stockpile.
- Finally, absence of a robust Advanced Development program placed too much risk on BioShield procurement programs.

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. The Department has established a framework to build medical countermeasure advanced development programs, critical to the evaluation of promising drug candidates and to their approval and licensure.

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 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 and contracts, primarily through the NIH. Procurement 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 PAHPA, the SRF payment was conditioned upon delivery of a product to the stockpile, and there were limited mechanisms 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 where a commercial market exists . Unfortunately, for biodefense medical products this stage has often proved challenging. The President's budget request included $189 million to help to fill this gap and support advanced development of promising biodefense product candidates in FY08.

BioShield Implementation

HHS recognizes that BioShield procurements must be made more swiftly. To help achieve this, HHS and the Department of Homeland Security (DHS) have established an interagency agreement to expedite the implementation of BioShield by clarifying roles and responsibilities and by establishing mechanisms to improve efficiencies. We have also secured direct funding for Project BioShield management and are building a highly qualified and accomplished workforce of acquisition specialists and scientists. Furthermore, HHS continues to seek ways to more effectively manage and streamline the time between the release of an RFP and the award of a contract

Because the process of product development can be fraught with unexpected complications and delays, it is nearly impossible to know the exact specifications for a product at the beginning of a Project BioShield acquisition. To address this challenge, HHS now requires that companies awarded Project BioShield contracts communicate with the Food and Drug Administration (FDA) early and often to ensure the success of each acquisition program.

Transparency and Outreach

The Department continues to work to make the BioShield process more transparent to stakeholders. The PHEMCE Strategy and Implementation Plan are the result of significant input from industry and other BioShield stakeholders, and provide insight into HHS countermeasure acquisition requirements and priorities. The PHEMCE Strategy and Implementation Plan reaffirms and further identifies our commitments to the development and acquisition of anthrax vaccines, anthrax antitoxins and therapeutics for radiological and nuclear threats. It also identifies the need for the continued development and acquisition of broad spectrum antibiotics, antivirals, and diagnostics against high priority threats. The end-to-end PHEMCE process has also increased our understanding of the challenging operational conditions that medical countermeasures must be designed for.

This outreach is critical to providing the visibility into BARDA programs necessary to ensure a mutual understanding between HHS and industry stakeholders, and to maximize participation. HHS is continually refining these processes 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

Thank you for the opportunity to present the progress HHS has made in national preparedness for biological threats to public health. We have made substantial progress. The threat remains real, and we have much left to do to ensure that we meet our mission of a Nation prepared for a public health emergency. This concludes my testimony. I will be happy to answer any questions.